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As filed with the Securities and Exchange Commission on January 2, 2014

Registration no. 333-

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**UNITED STATES  
SECURITIES AND EXCHANGE COMMISSION**  
Washington, D.C. 20549

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**Form F-1  
REGISTRATION STATEMENT  
UNDER  
THE SECURITIES ACT OF 1933**

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**UNIQUE B.V.\***

(Exact Name of Registrant as Specified in Its Charter)

**N/A**

(Translation of Registrant's Name into English)

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**The Netherlands**  
(State or Other Jurisdiction of  
Incorporation or  
Organization)

**2834**  
(Primary Standard Industrial  
Classification Code Number)

**Not applicable**  
(I.R.S. Employer  
Identification Number)

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**Approximate date of commencement of proposed sale to the public:** As soon as practicable after this Registration Statement becomes effective.

If any of the securities being registered on this Form are to be offered on a delayed or continuous basis pursuant to Rule 415 under the Securities Act of 1933, check the following box. ☐

If this Form is filed to register additional securities for an offering pursuant to Rule 462(b) under the Securities Act, check the following box and list the Securities Act registration statement number of the earlier effective registration statement for the same offering.    ☐

If this Form is a post-effective amendment filed pursuant to Rule 462(c) under the Securities Act, check the following box and list the Securities Act registration statement number of the earlier effective registration statement for the same offering.    ☐

If this Form is a post-effective amendment filed pursuant to Rule 462(d) under the Securities Act, check the following box and list the Securities Act registration statement number of the earlier effective registration statement for the same offering.    ☐

CALCULATION OF REGISTRATION FEE

TITLE OF EACH CLASS OF SECURITIES TO BE REGISTERED	PROPOSED MAXIMUM AGGREGATE OFFERING PRICE <sup>(1)(2)</sup>	AMOUNT OF REGISTRATION FEE
Ordinary shares, par value €0.01 per share	\$75,000,000	\$9,660

- (1)     Estimated solely for the purpose of determining the amount of registration fee in accordance with Rule 457(o) under the Securities Act of 1933.
- (2)     Calculated pursuant to Rule 457(o) based on an estimate of the proposed maximum aggregate offering price.

The Registrant hereby amends this Registration Statement on such date or dates as may be necessary to delay its effective date until the Registrant shall file a further amendment which specifically states that this Registration Statement shall thereafter become effective in accordance with Section 8(a) of the Securities Act of 1933 or until the Registration Statement shall become effective on such date as the Securities and Exchange Commission, acting pursuant to said Section 8(a), may determine.

\*

We intend to convert the legal form of our company under Dutch law from a private company with limited liability (*besloten vennootschap met beperkte aansprakelijkheid*) to a public company with limited liability (*naamloze vennootschap*) and to change our name from uniQure B.V. to uniQure N.V. prior to the consummation of this offering.

The information contained in this preliminary prospectus is not complete and may be changed. We may not sell these securities until the registration statement filed with the Securities and Exchange Commission is effective. This preliminary prospectus is not an offer to sell these securities and we are not soliciting an offer to buy these securities in any jurisdiction where the offer or sale is not permitted.

SUBJECT TO COMPLETION, DATED JANUARY 2, 2014

PRELIMINARY PROSPECTUS

Ordinary Shares

**uniQure**  
**uniQure B.V.**

We are offering \_\_\_\_\_ ordinary shares. This is our initial public offering, and no public market currently exists for our ordinary shares. We expect the initial public offering price to be between \$ \_\_\_\_\_ and \$ \_\_\_\_\_ per ordinary share. uniQure B.V. is a private company with limited liability (*besloten vennootschap met beperkte aansprakelijkheid*) incorporated under the laws of the Netherlands.

We have applied for listing of our ordinary shares on The NASDAQ Global Market under the symbol "QURE." We are an "emerging growth company" as defined by the Jumpstart Our Business Startups Act of 2012 and, as such, we have elected to comply with certain reduced public company reporting requirements for this prospectus and future filings.

Investing in our ordinary shares involves a high degree of risk. Please read "Risk Factors "beginning on page 15 of this prospectus.

Neither the Securities and Exchange Commission nor any state securities commission has approved or disapproved of these securities or determined if this prospectus is truthful or complete. Any representation to the contrary is a criminal offense.

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	PER ORDINARY SHARE	TOTAL
Public Offering Price	\$ _____	\$ _____
Underwriting Discounts and Commissions		
Proceeds to uniQure B.V., before Expenses		

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<sup>(1)</sup> The underwriters will also be reimbursed for certain expenses incurred in this offering. See "Underwriting" for details.

Delivery of the ordinary shares is expected to be made on or about \_\_\_\_\_, 2014. We have granted the underwriters an option for a period of 30 days to purchase an additional \_\_\_\_\_ ordinary shares. If the underwriters exercise their option in full, the total underwriting discounts and commissions payable by us will be \$ \_\_\_\_\_, and the total proceeds to us, before expenses, will be \$ \_\_\_\_\_.

**Jefferies**

**Leerink Swann**

**Piper Jaffray & Co.**

Prospectus dated \_\_\_\_\_, 2014.

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Neither we nor the underwriters have authorized anyone to provide you with any information other than that contained in this prospectus or in any free writing prospectus we may authorize to be delivered or made available to you. We take no responsibility for, and can provide no assurance as to the reliability of, any other information that others may give you. This prospectus may only be used where it is legal to offer and sell our ordinary shares. The information in this prospectus is accurate only as of the date of this prospectus, regardless of the time of delivery of this prospectus or any sale of our ordinary shares. Our business, financial condition, results of operations and prospects may have changed since that date. Neither we nor the underwriters are making an offer of these securities in any jurisdiction where the offer is not permitted.

Through and including \_\_\_\_\_, 2014 (25 days after the commencement of this offering), all dealers that buy, sell or trade our ordinary shares, whether or not participating in this offering, may be required to deliver a prospectus. This delivery requirement is in addition to the dealers' obligation to deliver a prospectus when acting as underwriters and with respect to their unsold allotments or subscriptions.

For investors outside the United States: Neither we nor the underwriters have done anything that would permit this offering or possession or distribution of this prospectus in any jurisdiction, other than the United States, where action for that purpose is required. Persons outside the United States who come into possession of this prospectus must inform themselves about, and observe any restrictions relating to, the offering of our ordinary shares and the distribution of this prospectus outside the United States.

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*Unless the context specifically indicates otherwise, references in this prospectus to "uniQure B.V.," "uniQure N.V.," "we," "our," "ours," "us," "our company" or similar terms refer to (1) uniQure B.V., together with its subsidiaries prior to our conversion into a public company with limited liability (naamloze vennootschap), and (2) uniQure N.V., together with its subsidiaries, after giving effect to our conversion into a public company with limited liability (naamloze vennootschap), which is expected to occur immediately prior to the consummation of this offering. The trademarks, trade names and service marks appearing in this prospectus are the property of their respective owners.*



## PROSPECTUS SUMMARY

*This summary does not contain all of the information you should consider before buying our ordinary shares. You should read the entire prospectus carefully, especially the "Risk Factors" section beginning on page 15, Management's Discussion and Analysis of Financial Condition and Results of Operations beginning on page 65 and our financial statements and the related notes appearing at the end of this prospectus, before deciding to invest in our ordinary shares.*

### Overview

We are a leader in the field of gene therapy and have developed the first and currently the only gene therapy product to receive regulatory approval in the European Union. Gene therapy offers the prospect of long-term and potentially curative benefit to patients with genetic or acquired diseases by directing the expression of a therapeutic protein or restoring the expression of a missing protein through a single administration. Our first product, Glybera, was approved by the European Commission in October 2012 under exceptional circumstances for the treatment of a subset of patients with lipoprotein lipase deficiency, or LPLD, a potentially life-threatening, orphan metabolic disease. We expect to launch Glybera commercially in selected European countries in the first half of 2014 through our collaboration with Chiesi Farmaceutici S.p.A., or Chiesi, which we entered into in April 2013. We retain full commercial rights to Glybera in the United States. In August and December 2013, we met with the Food and Drug Administration, or FDA, to discuss the regulatory pathway for Glybera in the United States, and we plan to file an Investigational New Drug application, or IND, with the FDA for Glybera in the first half of 2014.

We are developing a pipeline of additional gene therapies through multiple collaborations that are designed to accelerate the development and commercialization of these programs. We deliver our gene therapies through a delivery system, or vector, based on an engineered, non-replicating version of the adeno-associated virus, or AAV, one of several viruses commonly used as a vector in gene therapy. We develop our gene therapies using our innovative, modular technology platform, which consists of a suite of components that may be applied to multiple gene therapies and include our proprietary, cost-effective manufacturing process. Our pipeline includes product candidates targeting diseases for which either the efficacy of existing treatments is limited or the administration regimen is burdensome, such as hemophilia B, as well as diseases for which there are currently no treatments, such as Sanfilippo B syndrome. We initially intend to focus on orphan diseases but believe that we will also be able to leverage our technology to develop gene therapies targeting chronic and degenerative diseases that affect larger populations. Through our gene delivery know-how, our proprietary manufacturing process, the state-of-the-art facility we are building out in the United States, and our experience in developing and obtaining regulatory approval for Glybera in the European Union, we believe we will be able to develop and commercialize additional gene therapies more efficiently than our competitors.

### Our Gene Therapy Platform

Our gene therapy approach seeks to treat the causes of genetic diseases by enabling patients to effectively express a missing or deficient protein. To accomplish this, Glybera and our product candidates are designed to deliver a functional gene, or transgene, through a delivery system called a vector. Our approach is designed to be modular, in that it may allow us to efficiently develop, manufacture and seek regulatory approval for multiple gene therapies generally using the same principal components. The key components of our gene therapy approach are:

- **Therapeutic genes.** We design our gene therapies to deliver into the body's cells a transgene that encodes, or provides the blueprint for the expression of, a therapeutic protein. The transgene is carried in a gene cassette, or DNA sequence that encodes the specific gene, that includes DNA promoters that direct expression in specific tissues. We either develop our own gene cassettes or in-license them, often as part of our collaborations with academic research institutions and biotechnology and pharmaceutical companies. In-licensing gene cassettes provides us access to key

intellectual property and allows us to build upon our collaborators' scientific expertise and financial investment, as well as their preclinical and, in some cases, clinical development efforts.

- **AAV-based vector delivery system.** We deliver the gene cassette to the target tissue using an engineered, non-replicating viral vector delivery system based on AAV, a common virus that affects humans but does not cause disease. We believe that AAV is the vector of choice for most *in vivo* gene therapy applications, such as ours, in which the functional gene is introduced directly into the patient's body. We use different variants, or serotypes, of AAV, including AAV1, AAV2 and AAV5, each of which selectively targets particular tissues. In more than 80 gene therapy clinical studies conducted by us or third parties, AAV-based vectors raised no material safety concerns. AAV-based vectors have also demonstrated sustained expression in target tissues of animal models for more than ten years. In the hemophilia B Phase I/II clinical trial described below, St. Jude Children's Research Hospital in Memphis, Tennessee, or St. Jude, has reported expression in target tissue in humans for more than three years after a single treatment.
- **Administration technologies.** We and our collaborators are developing expertise in utilizing a variety of administration technologies to optimize the introduction of our gene therapy vectors to effectively deliver the transgene into the tissues and organs relevant to the indications we are targeting.
- **Scalable, proprietary manufacturing process.** We produce our AAV-based vectors in our own facilities with our proprietary manufacturing process, which uses insect cells and baculoviruses, a common family of viruses found in invertebrates. We believe that our manufacturing facility in Amsterdam, which the European Medicines Agency, or EMA, has approved for clinical and commercial grade production, and our facility near Boston, Massachusetts, which we are currently building out and equipping, will enable us to produce Glybera and other gene therapies cost-effectively at commercial scale.

## Our Competitive Strengths

Gene therapy has historically confronted a number of significant challenges, including safety concerns, limited efficacy, lack of commercially viable manufacturing technology and difficulties with effective administration. We believe we have overcome many of these challenges and have established integrated capabilities to support the clinical development and potential commercialization of our gene therapies. We believe that our key competitive strengths are the following:

- **A modular approach designed to enable us to develop gene therapies targeting multiple orphan diseases cost-effectively and on relatively short development timelines.** We expect that our modular approach will allow us to use the same building blocks to efficiently develop, manufacture and seek regulatory approval for multiple new gene therapies. In some cases, we believe that the disease-specific gene cassette will be the only component we need to change to target a new disease. As a result, we may be able to reduce the overall preclinical and potentially clinical development activities required to obtain regulatory approval, which may allow us to significantly reduce overall development risk, time and cost.
- **Experienced gene therapy research, clinical development and regulatory team.** We are applying the specialized research, clinical development and regulatory expertise we have acquired in developing and obtaining marketing authorization in the European Union for Glybera to develop additional gene therapies and navigate the complex regulatory process for gene therapies in other countries and for other product candidates. We have a team of more than 60 scientists and other experts, including 27 with Ph.D. or M.D. degrees or the foreign equivalent, with extensive experience in AAV-based gene therapy research and development.
- **Scalable, proprietary manufacturing process and facilities.** Our manufacturing process, which uses insect cells, is designed to produce higher yields of vectors more cost-effectively and efficiently than the mammalian cell-based approaches that many of our competitors utilize. We hold a non-exclusive license from the NIH for the use of baculoviruses and insect cells in the production of AAV-based vectors and have augmented this licensed technology with patented improvements to the replication

process designed to allow us to produce gene therapies at commercial scale. We have begun the build out of our 53,000 square foot manufacturing facility near Boston, Massachusetts, which we believe will be the world's largest dedicated, advanced production facility for AAV-based vectors. We believe that our manufacturing capabilities position us as a partner of choice for academic research institutions and biotechnology and pharmaceutical companies looking to bring AAV-based therapies into larger, late-stage clinical trials that require commercial scale processes.

- **Pioneering experience in gene therapy commercialization.** Gene therapy represents a potential shift in the paradigm of medical care with the commercialization challenges that often accompany a new approach. With our collaborator Chiesi, we are the first to initiate the market roll-out of an approved gene therapy in the European Union, including designing new models for product pricing and reimbursement based on a one-time intervention, expanding key opinion leader relationships, identifying centers of excellence, and developing physician and patient education and patient access programs. We believe our experience with Glybera in the European Union will facilitate our future efforts, subject to obtaining marketing approval, to commercialize Glybera and additional gene therapies in the United States and elsewhere.

## Glybera

Glybera is indicated for the treatment of adult patients diagnosed with familial LPLD confirmed by genetic testing and suffering from severe or multiple pancreatitis attacks despite dietary fat restrictions. We and our collaborator Chiesi are working to launch Glybera commercially in the European Union in the first half of 2014. We and Chiesi are developing a gene therapy pricing and business model for Glybera that is designed to capture the significant value we believe Glybera delivers to patients. We are also applying our experience in obtaining EMA approval of Glybera in the European Union to our development strategy in the United States. We met with the FDA in August and December 2013 to discuss the regulatory pathway in the United States for Glybera. If we receive regulatory approval from the FDA, we currently plan to market Glybera in the United States ourselves. We have begun preliminary preparations for a potential launch in the United States, including commissioning a third party pricing and reimbursement study and have conducted two market research studies directed at key opinion leaders. We have also initiated the development of a diagnostic referral program, engaged in key opinion leader and patient identification efforts and begun networking with key patient organizations in the United States.

LPLD is a serious, debilitating disease caused by mutations in the LPL gene, resulting in significantly diminished or absent activity of the LPL protein. LPLD results in hyper-chylomicronemia, or dramatic and potentially life-threatening increases in the level of large fat-carrying particles, called chylomicrons, in the blood after eating. In many cases, LPLD and the associated elevated levels of chylomicrons can cause acute and potentially life-threatening inflammation of the pancreas, known as pancreatitis, thus leading to frequent hospitalizations. Recurrent pancreatitis can lead to chronic abdominal pain, pancreatic insufficiency, which is an inability to properly digest food due to a lack of digestive enzymes made by the pancreas, and diabetes. Prior to Glybera, there was no approved therapy for LPLD. Patients are required to adhere to a strict low-fat diet and to abstain from alcohol. These restrictions, as well as the need for frequent hospitalizations and the constant fear of pancreatitis attacks, have a significant negative impact on the daily activity level of LPLD patients and on their quality of life.

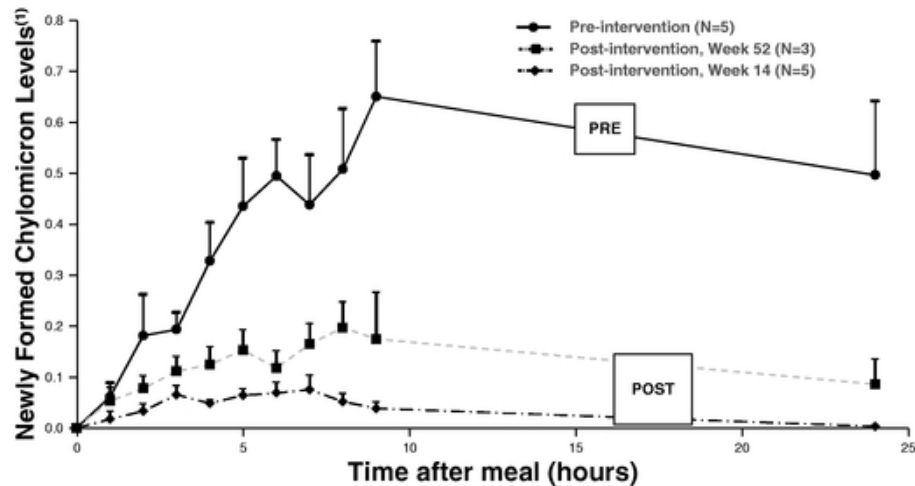
Glybera is designed to restore the lipoprotein lipase, or LPL, enzyme activity required by tissues of the body to clear, or process, the fat-carrying chylomicron particles that are formed in the intestine and transported via the blood to the muscle after a fat-containing meal. The product consists of an engineered copy of the human LPL gene packaged in a non-replicating AAV1 vector together with promoters that allow tissue-specific gene expression. AAV1 has a particular affinity, or tropism, for muscle cells.

As a basis for approval by the EMA, we conducted three open-label clinical trials in which we administered Glybera to a total of 27 LPLD patients. We also carried out a retrospective review of the clinical case notes for 17 of the 27 patients to determine the impact of Glybera treatment on the frequency and severity of

pancreatitis events. In this clinical program, the EMA concluded that Glybera had an acceptable overall safety profile based on a risk-benefit analysis.

In our third clinical trial of Glybera, involving five adult LPLD patients, we observed a consistent and significant improvement in the clearance of newly formed chylomicrons after a meal. The graph below depicts the appearance and removal of newly formed chylomicrons in the blood over a 24-hour period after a standardized meal. Patients were observed prior to treatment with Glybera, and at 14 weeks and 52 weeks following treatment. The top line in the graph represents the pretreatment condition and depicts mean levels of newly formed chylomicrons in the five patients prior to treatment with Glybera. We observed a consistent and significant improvement in the appearance and removal of newly formed chylomicrons in the blood in all five patients measured at week 14 after treatment, indicated by the bottom line in the graph, and all three patients measured at week 52 after treatment, indicated by the middle line in the graph.

#### Appearance and Removal of Newly Formed Chylomicrons Post-Meal



(1) Depiction of mean levels of newly formed chylomicrons using a radiolabeled tracer measured by tritium activity (in centimorgans). Participants consumed a standardized meal containing tritium-marked particles, which were measured in newly formed chylomicrons in the 24-hour period after the meal.

The case note review also provided evidence of clinical benefit in the form of a reduction of pancreatitis events and severity of attacks. Although these observations were made in a small number of patients for varying pre-treatment observation periods, and subject to statistical limitations, they suggested that Glybera leads to a clinically relevant reduction of pancreatitis risk in patients with severe or multiple pancreatitis attacks.

Recognizing that LPLD is an orphan condition, the EMA evaluated the totality of available quality, safety and efficacy data in considering our marketing authorization application for Glybera, including reviewing individual patient profiles. On the basis of that review, the EMA concluded that the benefit-risk balance of Glybera is favorable in the treatment of adult patients with familial LPLD diagnosed by genetic testing, with detectable levels of LPL protein and suffering from severe or multiple pancreatitis episodes despite dietary fat restrictions, and, therefore, recommended granting marketing authorization under exceptional circumstances. Marketing authorization under exceptional circumstances in the European Union is available for products for which the target indications are so rare that comprehensive data on efficacy and safety cannot reasonably be expected to be available prior to commercial launch. Prior to receiving this approval, our initial application for marketing approval for Glybera in the European Union was rejected in June 2011.

We requested a re-examination and, following further review, the EMA ultimately considered clinical benefit to be sufficiently established to allow for a positive benefit-risk estimation in an exceptional circumstances setting using a totality of the evidence approach.

To fulfill the key conditions of the approval of Glybera by the EMA, we are required to implement a patient registry prior to commercial launch and to complete an additional, post-approval clinical trial of Glybera, which we intend to commence in the first quarter of 2014. The principal goal of these programs will be to obtain additional data regarding the safety, efficacy and clinical benefit of Glybera. We also believe that these programs will help us to better define and target the LPLD patient population, as well as to raise awareness of LPLD and of Glybera in the clinician community.

In the European Union, we have been granted orphan drug exclusivity for Glybera for treatment of LPLD until October 2022, subject to the conditions applicable to orphan drug exclusivity. The FDA has also granted orphan drug designation to Glybera for treatment of LPLD.

In August and December 2013, we met with the FDA to discuss the regulatory pathway in the United States for Glybera. In contrast with the European Union, the United States does not have a process to approve marketing of a drug under exceptional circumstances. In our meetings, the FDA advised that it would require data in addition to what we had submitted to date to obtain marketing approval for Glybera in the European Union. The FDA recommended that we identify the clinical manifestations of LPLD for which Glybera might have the best prospects for demonstrating a meaningful impact in designing an adequate and appropriately controlled confirmatory trial. We may seek to amend the protocol for the European Union post-approval trial of Glybera described above so that such trial also could serve as such a confirmatory trial. In any event, we plan to file an IND with the FDA for Glybera in the first half of 2014 so that we can include United States LPLD patients in the post-approval trial. We also believe the patient registry described above that we are required to establish as part of our post-EU approval program will provide valuable data for the FDA to consider a part of the totality of our United States regulatory submissions. Our current expectation, subject to satisfactory completion of regulatory discussions with the FDA, is to have sufficient data from a confirmatory trial of Glybera and the patient registry described above to file a Biologics Licensing Application, or BLA, for Glybera with the FDA in late 2016 or 2017.

## Product and Development Pipeline

In addition to Glybera, our development pipeline includes our internal program for hemophilia B, two collaborator-sponsored programs for monogenic diseases, one collaborator-sponsored program for a chronic degenerative disease and several programs in early preclinical development. The following chart provides summary information on the most advanced of these programs:

Product / Product Candidate	Vector	Gene	Indication	Collaborator	Development Stage				Comments
					Pre-Clinical	Phase I / II	Phase II / III	Approved	
Internal Programs									
Glybera (E.U.)	AAV1	Lipoprotein Lipase (LPL)	LPLD	Chiesi	EU Commercial launch planned first half of 2014				• Post-approval study initiation in first quarter of 2014
Glybera (U.S.)	AAV1	LPL	LPLD	-	IND filing planned in first half of 2014				• Met with FDA in August and December 2013 to discuss regulatory pathway
Glybera (Rest of World)	AAV1	LPL	LPLD	-	Targeting markets that recognize EU marketing authorization				• Discussions with potential marketing collaborators ongoing
AMT-060	AAV5	Human Factor IX (hFIX) <sup>(1)</sup>	Hemophilia B	Chiesi					• Phase I/II trial by St. Jude using AAV5 & uniQure's hFIX transgene is ongoing • uniQure Phase I/II planned to commence second half of 2014
Collaborator Sponsored Programs									
AMT-021	AAV5	Porphobilinogen Deaminase <sup>(1)</sup>	Acute Intermittent Porphyria (AIP)	Digna Biotech (Licensor: CIMA)					• Phase I clinical trial by Digna Biotech ongoing
AMT-110	AAV5	NaGLU	Sanfilippo B Syndrome	Institut Pasteur (Sponsor: AFM)	Phase I/II commenced in October 2013				• Phase I/II clinical trial by Institut Pasteur commenced in October 2013
AAV2 Delivering GDNF <sup>(1)</sup>	AAV2	GDNF <sup>(1,2)</sup>	Parkinson's Disease	UCSF (Funder & Sponsor: NIH)					• Phase I trial by UCSF / NIH using AAV2 & GDNF transgene ongoing

internal programs

collaborator sponsored programs

third party trials using a uniQure transgene

- (1) hFIX, GDNF and PBGD transgenes have been exclusively licensed to uniQure.  
 (2) The trial commenced in May 2013; gene therapy was produced using mammalian-cell based process.

Below we provide further detail on our most advanced pipeline programs:

**Internal program: AMT-060 for hemophilia B.** In collaboration with Chiesi, we are developing AMT-060, a gene therapy for the treatment of hemophilia B, which is a severe blood clotting disorder that can lead to repeated and sometimes life-threatening episodes of external and internal bleeding. The current standard of care for the treatment of hemophilia B is prophylactic protein replacement therapy, requiring frequent intravenous administrations of human Factor IX, or hFIX, often costing approximately \$220,000 to \$340,000 per patient per year in the United States. We believe that the approximately 60% to 70% of the hemophilia B patient population who have either severe or moderately severe hemophilia would be eligible for treatment with gene therapy.

AMT-060 consists of an AAV5 vector carrying an hFIX transgene that we have exclusively licensed from St. Jude. We are currently conducting pre-IND toxicology animal studies of this product candidate. We plan to file an IND with the FDA and an Investigational Medicinal Product Dossier, or IMPD, with the EMA and then to initiate a Phase I/II, open label, dose escalation clinical trial of this product candidate in the second half of 2014 in 13 to 16 patients in Europe. We expect data from our clinical trial to be available in the second half of 2015.

St. Jude is currently conducting a Phase I/II, open label, dose escalation clinical trial in this indication with a gene therapy consisting of an AAV8 vector carrying the same therapeutic hFIX gene that we are using in AMT-060. In an article published in the *New England Journal of Medicine* in December 2011 reviewing interim data from six patients in the St. Jude clinical trial, the principal investigators reported that the vector used in the trial consistently led to long-term expression of the hFIX transgene at therapeutic levels in patients with severe hemophilia B, without acute or long-lasting toxicity. We understand from public presentations by the principal investigators for this trial that two additional patients at the highest dose level in this clinical trial have now also demonstrated such long-term expression. We believe that the interim results from this clinical trial constitute proof of concept of the use of this therapeutic gene in treating hemophilia B and may reduce the risks involved in our development of AMT-060.

**Collaborator-sponsored programs.** We are also collaborating with third parties that are sponsoring early-stage clinical trials of gene therapy product candidates to which we hold certain rights. We believe that this approach enables us to cost-effectively obtain access to preclinical and early-stage clinical results without expending significant resources of our own. These programs utilize either clinical materials that we have manufactured as part of our collaborations or gene cassettes that we have licensed. We generally have the rights to the data generated in these collaborator-sponsored clinical development programs, but do not control their design or timing. If we decide to progress any of these programs internally, we may need to develop or in-license additional technology. The most advanced of these programs are the following:

- **AMT-021 for Acute Intermittent Porphyria.** We and our collaborator Digna Biotech are developing AMT-021 as a gene therapy for acute intermittent porphyria, or AIP, a severe liver disorder. AMT-021 consists of an AAV5 vector carrying a therapeutic porphobilinogen deaminase, or PBGD, gene that we exclusively license from Applied Medical Research Center of the University of Navarra in Spain. Our collaborator Digna Biotech is currently conducting a Phase I clinical trial of AMT-021 in eight patients in Spain. We have manufactured the gene therapy being used in this clinical trial. We understand that, to date, Digna has not observed a reduction in the urinary levels of toxic metabolites in trial participants that could serve as a surrogate marker for efficacy. We believe that this result may suggest that a relative high level of transgene expression will be required for a gene therapy to provide a clinical benefit in this indication. We understand from Digna Biotech that data are expected in the second half of 2014. Under an agreement with Digna Biotech, we have an exclusive right to use all preclinical and Phase I clinical trial data from this program.
- **AMT-110 for Sanfilippo B Syndrome.** We and our collaborator Institut Pasteur are developing AMT-110 as a gene therapy for Sanfilippo B syndrome, a potentially fatal lysosomal storage disease that results in serious brain degeneration in children. This gene therapy consists of an AAV5 vector carrying a therapeutic  $\alpha$ -N-acetylglucosaminidase, or NaGLU, gene. Our collaborator Institut Pasteur is currently conducting a Phase I/II clinical trial of AMT-110 in four patients in France. We have manufactured the gene therapy being used in this clinical trial. We understand from Institut Pasteur that data are expected in the first half of 2015. We believe that if the results of this clinical trial are positive, it will constitute proof of concept of the administration to the brain of a gene therapy for lysosomal storage diseases.
- **AAV2/GDNF for Parkinson's Disease.** We and our collaborator the University of California at San Francisco, or UCSF, are developing gene therapy for Parkinson's disease, a progressive neurodegenerative disorder. UCSF is collaborating with the NIH to conduct a Phase clinical trial of a gene therapy in this indication consisting of an AAV2 vector carrying a therapeutic gene we have exclusively licensed in the gene therapy field from Amgen, Inc., or Amgen, that expresses a protein called glial cell line-derived neurotrophic factor, or GDNF. This clinical trial is being funded and sponsored by the NIH and will involve 24 patients. UCSF's product candidate has been manufactured by a third party using a mammalian cell-based process. In this clinical trial, the NIH is administering the gene therapy using convection enhanced delivery, which is a process developed by UCSF with the goal of achieving more precisely targeted administration than the methods used in early approaches, which may result in improved efficacy. We have a license under UCSF's rights to use all preclinical



and clinical data from the UCSF program for any future development program. Based on the results of the UCSF program, we may decide to develop an AAV2-based gene therapy containing the GDNF gene manufactured with our insect cell-based manufacturing process.

**Potential Additional Pipeline Programs.** We are also conducting early-stage preclinical research into a number of other potential applications of our technologies. Currently these programs focus on utilizing AAV5 in liver and CNS indications. Based on defined criteria for indications that we believe most likely to be well suited to our gene therapy approach, we have prioritized approximately ten additional target diseases. We may seek to develop these programs either independently or with collaborators who are already working in the relevant disease area, including collaborators that may have already conducted pre-clinical or clinical studies.

### Our Collaboration with Chiesi

We have entered into two agreements with Chiesi, a family-owned Italian pharmaceutical company with 2012 worldwide revenues of approximately €1.1 billion. One is an agreement for the commercialization of Glybera for LPLD and the second is an agreement for the co-development and commercialization of our hemophilia B program. We have retained full rights to United States, Canada and Japan under both agreements. We have received €17.0 million in aggregate upfront payments as well as a €14.0 million investment in our ordinary shares. In addition, these agreements provide us with research funding for further development of our hemophilia B product candidate, as well as the potential for commercial milestone payments of up to €42.0 million for Glybera for LPLD.

Under our Glybera commercialization agreement, we will receive payments from Chiesi for the quantities of Glybera we manufacture and supply to them. We are required to pay the cost of goods sold, including royalty and other payments to third parties in connection with the sale of Glybera. Based on our estimates, we anticipate we will retain in the range of 20% to 30% of the net sales of Glybera by Chiesi in the European Union and other countries under our agreement, net of the cost of goods sold, including the royalties and other obligations we owe to third parties. In addition, we are required to repay 20% of the gross amount received from Chiesi related to Glybera sales in repayment of a technical development loan from the Dutch government, which has a current outstanding balance of €5.4 million. Under our hemophilia B co-development agreement, we will also receive payments from Chiesi for any commercial quantities of our hemophilia product candidate we manufacture and supply to them, if we receive regulatory approval for such product candidate. We estimate that the amount we would retain, net of cost of goods sold, including third party royalties and related amounts, will be between 25% and 35% of the revenues from sales of such product by Chiesi, varying by country of sale.

### Our Strategy

Our strategic goal is to transform the paradigm of care for many severe and chronic diseases by moving from the short-term management of symptom to the potentially curative resolution of the disease through sustained therapeutic gene expression in target tissues. We are building on the capabilities that have enabled us to obtain the first regulatory approval of a gene therapy in the European Union to address a range of diseases for which we believe we can reach the market with a gene therapy ahead of our competitors. We seek to achieve our goal by pursuing the following key objectives:

- Maximize the value of Glybera.
- Exploit the potential of our gene therapy platform to develop AAV-based gene therapies for additional orphan monogenic diseases and selected chronic degenerative diseases.
- Leverage our competitive strengths to retain our position as a leading gene therapy company and establish additional collaborations.
- Continue to invest in our technology platform and expand our modular capabilities.



## Our Corporate Information

Our business was founded in 1998 and was initially operated through our predecessor company, Amsterdam Molecular Therapeutics (AMT) Holding N.V. or AMT. Following the initial rejection of our marketing authorization for Glybera in 2011, we undertook a corporate reorganization, pursuant to which the newly formed uniQure B.V. acquired the entire business and assets of AMT and completed a share-for-share exchange with the shareholders of AMT in the first half of 2012. We intend to re-register as a public limited company in the Netherlands in connection with this offering. Our executive offices are located at Meibergdreef 61, Amsterdam 1105 BA, the Netherlands, and our telephone number is +31 20 566 7394. Our website address is [www.uniqure.com](http://www.uniqure.com). The information contained on, or accessible through, our website is not a part of this prospectus.

## Risk Associated with Our Business

Our business is subject to a number of risks of which you should be aware before making an investment decision. These risks are discussed more fully in the "Risk Factors" section of this prospectus. These risks include the following:

- We have a history of operating losses and anticipate that we will continue to incur losses for the foreseeable future. As of September 30, 2013, we had an accumulated deficit of €138.0 million. We will likely need additional funding, and such funding may cause substantial dilution to our shareholders.
- Our financial prospects and ability to generate revenues for the next several years depend heavily on the successful commercialization of Glybera in the European Union in collaboration with our partner Chiesi, and upon our ability to obtain additional marketing approvals and ultimately commercialize Glybera in the United States and other countries, which may not occur for several years, if ever. To obtain marketing approval for Glybera in the United States, we will need to successfully conduct an adequate and appropriately controlled confirmatory clinical trial either as part of the EMA-mandated post-approval clinical trial or separately, to obtain data needed to file a BLA for Glybera with the FDA.
- As gene therapies, Glybera and our product candidates are novel technologies and face uncertainty in the regulatory review and approval process. We cannot predict when or if we will obtain marketing approval to commercialize a product candidate, and any approval we may receive may be for a narrower indication than we expect or may be subject to costly post-approval requirements, which could restrict or eliminate the potential commercial success of the product candidate.
- Our product candidates are in early clinical or preclinical development and there is significant risk of failure or delay in these programs. We rely on our collaborators for important aspects of our development program and in many cases we have limited or no control over the design and conduct of the trials our collaborators conduct, or the efforts and resources our collaborators expend.
- The future growth of our business depends in significant part on our ability to enter into in-licenses or acquire rights to new product candidates and technologies, and to enter into additional collaborations in the future. If we are unable to attract collaborators or successfully identify or compete for the rights to new technologies, our prospects for growth could suffer.
- If we fail to obtain or sustain adequate prices and reimbursement for Glybera and other product candidates for which we may receive marketing approval, our ability to market and sell our products would be adversely affected and our financial position would suffer.
- We may be unable to obtain, maintain and protect necessary intellectual property assets, which could harm our ability to compete and impair our business. We are heavily reliant upon licenses of proprietary technology from third parties and these licenses may not provide adequate rights, we may lose or be unable to protect these rights, or we may be unable to acquire additional intellectual property required for our development programs.

- We face substantial competition, and others may discover, develop or commercialize products before or more successfully than we do.

#### **Implications of Being an Emerging Growth Company**

We qualify as an "emerging growth company" as defined in the Jumpstart our Business Startups Act of 2012, or the JOBS Act. An emerging growth company may take advantage of specified reduced reporting and other burdens that are otherwise applicable generally to public companies. As an emerging growth company, we are electing to take advantage of the following exemptions:

- providing two years rather than three years of audited financial statements;
- not providing an auditor attestation report on our system of internal control over financial reporting; and
- not complying with any requirement that may be adopted by the Public Company Accounting Oversight Board regarding mandatory auditor firm rotation or a supplement to the auditor's report providing additional information about the audit and the financial statements (auditor discussion and analysis).

The JOBS Act permits an "emerging growth company" such as us to take advantage of an extended transition period to comply with new or revised accounting standards applicable to public companies. We are irrevocably electing not to avail ourselves of this extended transition period for complying with new or revised accounting standards and, therefore, we will be subject to the same new or revised accounting standards as other public companies that are not "emerging growth companies."

We may take advantage of these exemptions for up to five years or such earlier time as we are no longer an emerging growth company. We would cease to be an emerging growth company if we have more than \$1.0 billion in annual revenues, have more than \$700 million in market value of our ordinary shares held by non-affiliates or issue more than \$1.0 billion of non-convertible debt over a three-year period.

## THE OFFERING

Ordinary shares offered by us:	ordinary shares
Ordinary shares to be outstanding immediately after this offering:	ordinary shares
Offering price	The initial public offering price per ordinary share is expected to be between \$                      and \$                      .
Listing	We have applied for listing of our ordinary shares on the NASDAQ Global Market under the symbol "QURE."
Option to purchase additional shares	We have granted to the underwriters an option, which is exercisable within 30 days from the date of this prospectus, to purchase an aggregate of up to an additional                      ordinary shares. See "Underwriting" for more information.
Use of proceeds	<p>We currently estimate that we will use the net proceeds from this offering, together with our cash on hand, as follows:</p> <ul style="list-style-type: none"> <li>• to complete the building out and equipping of our manufacturing facility in Lexington, Massachusetts;</li> <li>• to support our further clinical development of Glybera, and our application for marketing approval of Glybera and preparation for potential commercial launch in the United States;</li> <li>• to fund our planned Phase I/II clinical trial of AMT-060 in hemophilia B; and</li> <li>• to advance the development of our other product candidates, for working capital and for general corporate purposes, including service on our indebtedness and possibly acquisitions or investments in other businesses, technologies or product candidates.</li> </ul> <p>See "Use of Proceeds" for additional information.</p>
Risk factors	See "Risk Factors" and other information included in this prospectus for a discussion of risks you should carefully consider before investing in our ordinary shares.

The total number of ordinary shares that will be outstanding immediately after this offering includes:

- an aggregate of 60,974,570 ordinary shares to be outstanding immediately prior to the closing of this offering; and
- ordinary shares to be issued and sold by us in this offering;

and excludes:

- 8,451,110 ordinary shares issuable upon the exercise of options outstanding as of September 30, 2013 at a weighted average exercise price of €0.78 per share. See "Management—Compensation—Share Options;"
- up to                      ordinary shares reserved for future issuance under our equity incentive plans following this offering; and
- 854,036 ordinary shares issuable upon the exercise of warrants outstanding as of September 30, 2013 at an exercise price of €2.02 per share.

Unless otherwise indicated, all information in this prospectus assumes:

- that the underwriters do not exercise their option to purchase an aggregate of up to an additional                      ordinary shares from us;
- our conversion into a public limited company with limited liability (*naamloze vennootschap*) under the laws of the Netherlands and amendment of our articles of association, which will occur before the completion of this offering;
- the conversion of our class A, class B and class C ordinary shares into an aggregate of 60,974,570 ordinary shares, which will occur prior to completion of this offering; and
- the                      -for-                      consolidation of our ordinary shares, which will have the effect of a reverse share split, which will occur prior to completion of this offering.

## SUMMARY CONSOLIDATED FINANCIAL DATA

The following summary consolidated financial data as of and for the years ended December 31, 2011 and 2012 have been derived from our consolidated financial statements included elsewhere in this prospectus. The following summary consolidated financial data as of and for the nine months ended September 30, 2012 and 2013 have been derived from our unaudited consolidated financial statements included elsewhere in this prospectus. The unaudited financial statements have been prepared on the same basis as the audited financial statements and, in the opinion of management, reflect all adjustments, which include only normal recurring adjustments, necessary to state fairly our financial position as of September 30, 2013 and the results of operations for the nine months ended September 30, 2012 and 2013. The summary consolidated financial data below should be read together with those consolidated financial statements as well as the "Selected Consolidated Financial Data" and the "Management's Discussion and Analysis of Financial Condition and Results of Operations" sections of this prospectus. Our historical results for any prior period are not necessarily indicative of results to be expected in any future period, and our interim period results are not necessarily indicative of results to be expected for a full year or any other interim period.

### Consolidated Statements of Comprehensive Income Data:

€ in thousands (except per share data)	YEAR ENDED DECEMBER 31,		NINE MONTHS ENDED SEPTEMBER 30,	
	2011	2012	2012	2013
<b>Revenues:</b>				
License revenues	—	—	—	€ 220
Collaboration revenues	—	—	—	1,831
<b>Total revenues</b>	—	—	—	<b>2,051</b>
Cost of goods sold	—	—	—	(800)
<b>Gross profit</b>	—	—	—	<b>1,251</b>
Other income	€ 2,192	€ 649	€ 315	686
Research and development expenses	(15,500)	(10,231)	(5,690)	(9,856)
Selling, general and administrative expenses	(3,807)	(4,564)	(4,438)	(7,612)
Other losses, net	(26)	(45)	(82)	(269)
<b>Operating result</b>	<b>(17,141)</b>	<b>(14,191)</b>	<b>(9,895)</b>	<b>(15,800)</b>
Finance income	277	22	16	48
Finance expense	(436)	(547)	(545)	(4,676)
<b>Net loss</b>	<b>(17,300)</b>	<b>(14,716)</b>	<b>(10,424)</b>	<b>(20,428)</b>
<b>Basic and diluted loss per share</b>	<b>(0.73)</b>	<b>(0.34)</b>	<b>(0.25)</b>	<b>(0.39)</b>
Weighted average shares outstanding used in computing per share amounts:				
Basic and diluted	23,549	43,187	42,156	52,972

The following table summarizes our balance sheet data as of September 30, 2013:

- on an actual basis; and
- on an as adjusted basis to give effect to the sale of ordinary shares by us in this offering, assuming an initial public offering price of \$ per ordinary share share, the midpoint of the price range set forth on the cover page of this prospectus, and after deducting the estimated underwriting discounts and commissions and estimated offering expenses payable by us and the application of such net proceeds as described under "Use of Proceeds."

**Consolidated Balance Sheet Data:**

(€ in thousands)	AS OF SEPTEMBER 30, 2013	
	ACTUAL	AS ADJUSTED <sup>(1)</sup>
Cash and cash equivalents	€ 31,427	€
Total assets	43,671	
Total debt	8,456	
Accumulated deficit	(137,656)	
Total shareholders' equity (deficit)	11,321	

- <sup>(1)</sup> Each \$1.00 increase or decrease in the assumed initial public offering price of \$ per ordinary share would increase or decrease, respectively, the amount of cash and cash equivalents, working capital, total assets and total shareholders' equity by \$ million, assuming the number of ordinary shares offered by us, as set forth on the cover page of this prospectus, remains the same and after deducting the underwriting discounts and commissions and estimated offering expenses payable by us. We may also increase or decrease the number of ordinary shares we are offering. An increase or decrease of 1,000,000 in the number of ordinary shares we are offering would increase or decrease, respectively, the amount of cash, cash equivalents and short-term investments, working capital, total assets and stockholders' equity by approximately \$ million, assuming the assumed initial public offering price per ordinary share, as set forth on the cover page of this prospectus, remains the same. The as adjusted information is illustrative only, and we will adjust this information based on the actual initial public offering price and other terms of this offering determined at pricing.

## RISK FACTORS

*Investing in our ordinary shares involves a high degree of risk. You should carefully consider the risks and uncertainties described below together with all of the other information contained in this prospectus, including our financial statements and the related notes appearing at the end of this prospectus, before deciding to invest in our ordinary shares. If any of the following risks actually occurs, our business, prospects, operating results and financial condition could suffer materially. In such event, the trading price of our ordinary shares could decline and you could lose part or all of your investment.*

### Risks Related to Our Financial Position and Need for Additional Capital

***We have incurred significant losses to date, expect to incur losses over the next several years and may never achieve or maintain profitability.***

We have incurred significant losses to date. We had a net loss of €20.4 million in the first nine months of 2013, €14.7 million in 2012 and €17.3 million in 2011. As of September 30, 2013, we had an accumulated deficit of €138.0 million. To date, we have financed our operations primarily through the sale of equity securities and convertible debt and, to a lesser extent, through milestone payments, subsidies and grants from governmental agencies and fees for services. We have devoted substantially all of our financial resources and efforts to research and development, including preclinical studies and clinical trials. Our product, Glybera, received marketing approval under exceptional circumstances from the European Commission in October 2012. We plan in the future to apply for marketing approval for Glybera in the United States and other countries and expect that we will be required to conduct one or more additional clinical trials of Glybera. We are still in the early stages of development of the other product candidates in our pipeline. We expect to continue to incur significant expenses and losses over the next several years. Our net losses may fluctuate significantly from quarter to quarter and year to year. We anticipate that our expenses will increase substantially as we:

- complete the EMA-mandated post-approval clinical trial of Glybera and implementation of an LPLD patient registry;
- conduct a confirmatory clinical trial of Glybera, either as part of the EMA-mandated post-approval clinical trial or separately, to obtain data needed to file a BLA for Glybera with the FDA;
- seek marketing approval of Glybera in the United States and other countries;
- initiate a Phase I/II clinical trial of AMT-060 for hemophilia B in collaboration with Chiesi;
- advance the clinical development of our other product candidates, most of which are at early stages of development, and seek to discover and develop additional product candidates;
- seek marketing approval for any product candidates that successfully complete clinical trials;
- establish a sales, marketing and medical affairs infrastructure in the United States;
- continue the build-out of our manufacturing facility in Lexington, Massachusetts to expand our manufacturing capabilities for Glybera and our pipeline of product candidates;
- maintain, expand and protect our intellectual property portfolio, including in-licensing additional intellectual property rights from third parties;
- hire additional personnel, particularly in our manufacturing, research, clinical development, medical affairs, commercial and quality control groups; and
- add operational, financial and management information systems and related finance and compliance personnel necessary to operate as a public company.

We are only in the preliminary stages of most of these activities. We and our collaborators may never succeed in these activities and, even if we do, may never generate revenues that are significant enough to achieve profitability.

Even if we do achieve profitability, we may not be able to sustain or increase profitability. Our failure to become and remain profitable would depress the value of our company and could impair our ability to expand our business, maintain our research and development efforts, diversify our product offerings or even continue our operations. A decline in the value of our company could also cause you to lose part or all of your investment.

***Our financial results will substantially depend on the commercial success of sales of Glybera.***

We anticipate that our collaborator Chiesi will commercially launch Glybera in the European Union in the first half of 2014 and that revenues from sales of Glybera will be one of the principal sources of funds for our business for at least the next several years. Because our business is currently dependent on Glybera, failure to achieve anticipated revenues from this product would have an adverse effect on our results of operations and cause the value of our ordinary shares to decline. A number of factors, some of which are out of our control, may adversely affect the commercial success of Glybera, including the following:

- our collaborator Chiesi may not successfully commercialize Glybera in the European Union and other specified countries in the Chiesi territory;
- the post-approval requirements imposed by the EMA in connection with Glybera's approval under exceptional circumstances may be costly or may eventually lead to withdrawal of approval;
- we may never be able to obtain marketing approval for Glybera in the United States or other countries;
- Glybera may fail to achieve market acceptance by physicians, patients, third party payors and others in the medical community;
- other alternative treatments for LPLD may be developed and gain commercial acceptance, eroding Glybera's market share;
- the limited label we have received for Glybera in the European Union may limit our addressable market, and other regulatory agencies may approve Glybera only with a similarly limited label;
- we may be unable to establish or maintain sales, marketing and medical affairs capabilities for the commercialization of Glybera in the United States, even if we receive FDA approval; and
- coverage, pricing and reimbursement levels may be lower than we expect.

***Even if our commercialization of Glybera or other product candidates for which we obtain marketing approval is successful, we may not be financially successful due to our obligations to third parties.***

We have obtained exclusive or non-exclusive rights from third parties under a range of patents and other technology that we are exploiting in Glybera and our development programs. Our agreements with these third parties generally grant us a license to make, use, sell, offer to sell and import products covered by the licensed patent rights in exchange for our payment of some combination of an upfront amount, annual fees, royalties, a portion of amounts we receive from our sublicensees and payments upon the achievement of specified development, regulatory or commercial milestones. For example, we are contractually obligated to pay royalties and other obligations to third parties on net sales of Glybera by us, Chiesi or other sublicensees or on other amounts we receive, including from Chiesi or other sublicensees for their sales of Glybera. We also received a technical development loan from the Dutch government, which requires repayment based on the timing and amount of revenues we receive from the sale of Glybera. These financial obligations to third parties are an expense to us, which could adversely affect our financial position.



***We will likely need to raise additional funding, particularly if we experience delays in implementing our development programs or commercialization efforts. Additional funding may not be available on acceptable terms, or at all, and any failure to obtain capital when needed may force us to delay, limit or terminate our product development efforts or other operations.***

We expect to incur significant expenses in connection with our ongoing activities and expect that we will likely need to obtain substantial additional funding in connection with our continuing operations. We have based our estimate of our financing requirements on assumptions that may prove to be wrong, and we could use our capital resources sooner than we currently expect. Our future capital requirements will depend on many factors, including:

- the commercial success of Glybera, including the timing and amount of revenues generated, as well as our cost of goods sold;
- our collaboration agreements remaining in effect and our ability to obtain research and development funding and achieve milestones under these agreements;
- the progress and results of our current and planned clinical trials and those of our collaborators;
- the scope, progress, results and costs of preclinical development, laboratory testing and clinical trials for our additional product candidates;
- the number and development requirements of other product candidates that we pursue;
- the cost, timing and outcome of regulatory review of our product candidates, particularly for approval of Glybera in the United States;
- the cost and timing of future commercialization activities by us or our collaborators, including product manufacturing, marketing, sales and distribution, for Glybera and any of our product candidates for which we receive marketing approval in the future;
- the amount and timing of revenues, if any, we receive from commercial sales of any product candidates for which we receive marketing approval in the future;
- the costs and timing of preparing, filing and prosecuting patent applications, maintaining and enforcing our intellectual property rights and defending any intellectual property-related claims;
- the extent to which we acquire or in-license other products or technologies; and
- the cost and progress of the build-out of our Lexington, Massachusetts manufacturing facility.

Adequate capital may not be available to us when needed or may not be available on acceptable terms. Further, our ability to obtain debt financing may be limited by covenants we have made under our Loan and Security Agreement with Hercules Technology Growth Capital, Inc., or Hercules, and our pledge to Hercules of substantially all of our assets as collateral. These covenants, with certain exceptions, limit the ability of the Company to incur additional indebtedness and liens, pay dividends, make acquisitions, or sell or dispose of property and assets. Additionally, they require us to maintain cash equivalents on deposit in the United States of at least the lesser of (1) 100% of the then outstanding principal amount or (2) 50% of all the worldwide cash and cash equivalents. If we are unable to raise capital when needed or on attractive terms, we could be forced to delay, reduce or eliminate our research and development programs or any future commercialization efforts and would have a negative impact on our financial condition.

In addition, we may wish to seek additional capital due to favorable market conditions or strategic considerations, even if we believe we have sufficient funds for our current or future operating plans.

***Raising additional capital may cause dilution to our shareholders, including purchasers in this offering, restrict our operations or require us to relinquish rights to our technologies or product candidates.***

To the extent that we raise additional capital through the sale of equity or convertible debt securities, your ownership interest will be diluted, and the terms of these securities may include liquidation or other preferences that adversely affect your rights as an ordinary shareholder. Debt financing and preferred equity financing, if available, may involve agreements that include covenants limiting or restricting our ability to take specific actions, such as incurring additional debt, making capital expenditures or declaring dividends.

If we raise additional funds through collaborations, strategic alliances or marketing, distribution or licensing arrangements with third parties, we may have to relinquish valuable rights to our technologies, future revenue streams, products or product candidates or grant licenses on terms that may not be favorable to us. If we are unable to raise additional funds through equity or debt financings when needed, we may be required to delay, limit, reduce or terminate our product development or future commercialization efforts or grant rights to develop and market products or product candidates that we would otherwise prefer to develop and market ourselves.

***Our existing and any future indebtedness could adversely affect our ability to operate our business.***

As of September 30, 2013, we had recognized a liability of €7.3 million (\$9.8 million) of outstanding borrowings under our Loan and Security Agreement with Hercules, which we are required to repay in monthly installments through October 1, 2016. We do not intend to use the net proceeds of this offering to prepay these obligations. We could in the future incur additional debt obligations beyond our borrowings from Hercules. Our existing loan obligations, together with other similar obligations that we may incur in the future, could have significant adverse consequences, including:

- requiring us to dedicate a portion of our cash resources to the payment of interest and principal, reducing money available to fund working capital, capital expenditures, product development and other general corporate purposes;
- increasing our vulnerability to adverse changes in general economic, industry and market conditions;
- subjecting us to restrictive covenants that may reduce our ability to take certain corporate actions or obtain further debt or equity financing;
- limiting our flexibility in planning for, or reacting to, changes in our business and the industry in which we compete; and
- placing us at a competitive disadvantage compared to our competitors that have less debt or better debt servicing options.

We may not have sufficient funds, and may be unable to arrange for additional financing, to pay the amounts due under our existing loan obligations. Failure to make payments or comply with other covenants under our existing debt could result in an event of default and acceleration of amounts due. Under our agreement with Hercules, the occurrence of an event that would reasonably be expected to have a material adverse effect on our business, operations, assets or condition is an event of default. If an event of default occurs and the lender accelerates the amounts due, we may not be able to make accelerated payments, and the lender could seek to enforce security interests in the collateral securing such indebtedness, which includes substantially all of our assets. In addition, the covenants under our existing debt, and the pledge of our assets as collateral, could limit our ability to obtain additional debt financing.

***Our business operations may be negatively affected by the strategic restructuring we undertook in 2012.***

At the end of 2011, following the initial rejection of our application for marketing approval for Glybera in the European Union, our predecessor entity, Amsterdam Molecular Therapeutics, or AMT, initiated a strategic restructuring in order to conserve resources and improve its financial position. As part of this effort, AMT significantly reduced personnel, programs and spending. As a result, we lost many talented employees, including employees with an extensive understanding of our clinical programs as well as our regulatory and financial affairs. In the fourth quarter of 2011, total staff was reduced from 92 to 49. Since that time, we have hired a number of new staff, and total headcount as of September 30, 2013 was 76. In addition, we have engaged 33 consultants and contract workers. Nevertheless, this loss of talent and institutional knowledge has adversely affected our operations during the past year and may result in delays in preparing regulatory filings, completing clinical trials and other related activities, and could negatively impact our future business operations.

## Risks Related to the Development of Our Product Candidates

### ***We may not be successful in our efforts to use our gene therapy technology platform to build a pipeline of additional product candidates.***

A key element of our strategy is to use our gene therapy technology platform to expand our pipeline of gene therapies and to progress these product candidates through clinical development together with our collaborators. Although we currently have a pipeline of programs at various stages of development, we may not be able to identify or develop product candidates that are safe and effective. Even if we are successful in continuing to build our pipeline, the potential product candidates that we identify may not be suitable for clinical development, including as a result of being shown to have harmful side effects or other characteristics that indicate that they are unlikely to receive marketing approval and achieve market acceptance. Research programs to identify new product candidates require substantial technical, financial and human resources. We or our collaborators may focus our efforts and resources on potential programs or product candidates that ultimately prove to be unsuccessful. If we do not continue to successfully develop and commercialize product candidates based upon our technology, we may face difficulty in obtaining product revenues in future periods, which could result in significant harm to our financial position and adversely affect our share price.

### ***Our strategy of obtaining rights to key technologies through in-licenses may not be successful.***

We seek to expand our product pipeline in part by in-licensing the rights to key technologies, including those related to gene delivery and gene cassettes. The future growth of our business will depend in significant part on our ability to in-license or otherwise acquire the rights to additional product candidates or technologies, particularly through our collaborations with academic research institutions. However, we may be unable to in-license or acquire the rights to any such product candidates or technologies from third parties on acceptable terms or at all. The in-licensing and acquisition of these technologies is a competitive area, and a number of more established companies are also pursuing strategies to license or acquire product candidates or technologies that we may consider attractive. These established companies may have a competitive advantage over us due to their size, cash resources and greater clinical development and commercialization capabilities. In addition, companies that perceive us to be a competitor may be unwilling to license rights to us. Furthermore, we may be unable to identify suitable product candidates or technologies within our area of focus. If we are unable to successfully obtain rights to suitable product candidates or technologies, our business, financial condition and prospects could suffer.

### ***We may encounter substantial delays in our clinical trials or fail to demonstrate the safety and efficacy of our product candidates.***

Clinical development is expensive, time-consuming and uncertain as to outcome. Our product candidates are in early clinical or preclinical development, and there is a significant risk of failure or delay in each of these programs. In several of our programs, we intend to transition a collaborator's program to a different viral vector or to our insect-cell based manufacturing process, which could result in additional development challenges and delays. We cannot guarantee that any clinical trials will be conducted as planned or completed on schedule, if at all. A failure of one or more clinical trials can occur at any stage of testing. Events that may prevent successful or timely completion of clinical development include:

- delays in reaching a consensus with regulatory agencies on study design;
- delays in reaching agreement on acceptable terms with prospective clinical research organizations, or CROs, and clinical trial sites;
- delays in obtaining required Institutional Review Board, or IRB, approval at each clinical trial site;
- delays in recruiting suitable patients to participate in our clinical trials, particularly due to the rare nature of many of our target orphan monogenic diseases;
- imposition of a clinical hold by regulatory agencies after an inspection of our clinical trial operations or trial sites;

- failure by CROs, other third parties or us to adhere to clinical trial requirements;
- failure to perform in accordance with the FDA's good clinical practices, or GCP, or applicable regulatory guidelines in other countries;
- delays in the testing, validation, manufacturing and delivery of our product candidates to the clinical sites;
- delays in having patients complete participation in a study or return for post-treatment follow-up;
- clinical trial sites or patients dropping out of a study;
- occurrence of serious adverse events associated with a product candidate that are viewed to outweigh its potential benefits; or
- changes in regulatory requirements and guidance that require amending or submitting new clinical protocols.

Any inability to successfully complete preclinical and clinical development could result in additional costs to us or impair our ability to generate revenues from product sales, regulatory and commercialization milestones and royalties. In addition, if we make manufacturing or formulation changes to our product candidates, including changes in the vector or manufacturing process used, we may need to conduct additional studies to bridge our modified product candidates to earlier versions. Clinical trial delays could also shorten any periods during which we may have the exclusive right to commercialize our product candidates or allow our competitors to bring products to market before we do, which could impair our ability to successfully commercialize our product candidates and may harm our business and results of operations.

***If clinical trials of our product candidates fail to satisfactorily demonstrate safety and efficacy to regulatory authorities, we may incur additional costs or experience delays in completing, or ultimately be unable to complete, the development and commercialization of our product candidates.***

Before obtaining marketing approval from regulatory authorities for the sale of our product candidates, we must conduct extensive clinical trials to demonstrate the safety and efficacy of the product candidates in humans. It is impossible to predict when or if any of our product candidates will prove effective or safe in humans or will receive marketing approval. If the results of our clinical trials are inconclusive or if there are safety concerns or adverse events associated with our product candidates, we may:

- be delayed in or altogether prevented from obtaining marketing approval for our product candidates;
- obtain approval for indications or patient populations that are not as broad as intended or desired;
- obtain approval with labeling that includes significant use or distribution restrictions or safety warnings;
- be subject to changes with the way the product is administered;
- be required to perform additional clinical trials to support approval or be subject to additional post-marketing testing requirements;
- have regulatory authorities withdraw their approval of the product or impose restrictions on its distribution in the form of a modified risk evaluation and mitigation strategy;
- be subject to the addition of labeling statements, such as warnings or contraindications;
- be sued; or
- experience damage to our reputation.

Any of these events could prevent us from achieving or maintaining market acceptance of our product candidates and impair our ability to commercialize our products.

***We may experience delays or difficulties in the enrollment of patients in clinical trials, particularly for orphan indications, which may delay or prevent our receipt of necessary regulatory approvals.***

Identifying and qualifying patients to participate in clinical trials of our product candidates is critical to our success. The timing of our clinical trials and those of our collaborators depends on the speed at which we or they can recruit patients to participate in such trials. We or our collaborators may not be able to locate and enroll a sufficient number of eligible patients to participate in these trials as required by the FDA, the EMA or similar regulatory authorities outside the United States and the European Union. In particular, because several of our programs are focused on the treatment of patients with orphan diseases, our ability to enroll eligible patients in these trials may be limited or slower than we anticipate in light of the small patient populations involved. For example, we reduced the number of patients enrolled in our second Phase II/III clinical trial of Glybera from the 16 patients originally planned to five patients due to slow recruitment. In addition, our potential competitors, including major pharmaceutical, specialty pharmaceutical and biotechnology companies, academic institutions and governmental agencies and public and private research institutions, may seek to develop competing therapies, which would further limit the small patient pool available for our studies. Patient enrollment may be affected by other factors including:

- the severity of the disease under investigation;
- design of the study protocol;
- the eligibility criteria for the study;
- the perceived risks, benefits and convenience of administration of the product candidate being studied;
- our efforts to facilitate timely enrollment in clinical trials;
- the patient referral practices of physicians; and
- the proximity and availability of clinical trial sites to prospective patients.

An inability by us or our collaborators to locate and enroll a sufficient number of patients for clinical trials may result in our failure to initiate or continue clinical trials for our product candidates, or may cause significant delays in such trials and could require us or our collaborators to abandon one or more clinical trials altogether. Enrollment delays may also result in increased development costs for our product candidates, which could cause the value of our company to decline.

***Our progress in early-stage clinical trials may not be indicative of long-term efficacy in late-stage clinical trials, and our progress in trials for one product candidate may not be indicative of progress in trials for other product candidates.***

With the exception of Glybera, the product candidates in our pipeline are at early-stages of development. Study designs and results from previous studies are not necessarily predictive of our future clinical study designs or results, and initial results may not be confirmed upon full analysis of the complete study data. Our product candidates may fail to show the desired safety and efficacy in later stages of clinical development despite having successfully advanced through initial clinical studies.

A number of companies in the pharmaceutical and biotechnology industries have suffered significant setbacks in late-stage clinical trials even after achieving promising results in early-stage clinical trials. If a larger population of patients does not experience positive results, if these results are not reproducible, or if our products show diminishing activity over time, our products may not receive approval from the EMA or FDA. Data obtained from preclinical and clinical activities are subject to varying interpretations, which may delay, limit or prevent regulatory approval. In addition, we may encounter regulatory delays or rejections as a result of many factors, including changes in regulatory policy during the period of product development. Failure to confirm favorable results from earlier trials by demonstrating the safety and effectiveness of our products in late stage clinical trials with larger patient populations could have a material adverse effect on our business that would cause our share price to decline.

Progress in trials of Glybera and its approval in the European Union do not indicate that we will make similar progress in trials for our other product candidates. While Glybera uses an AAV1 vector for gene delivery, the rest of the product candidates in our pipeline use other AAV vector variants, such as AAV5 or AAV2. Also, while Glybera is injected directly into the muscles of the leg, the rest of the products in our pipeline target other tissues. Due to these variations, trials for our other product candidates may be less successful than the trials for Glybera.

***If serious adverse events occur or unacceptable side effects are identified in any gene therapy products or product candidates, whether ours or those of our competitors, we may need to abandon or limit the sale or development of Glybera or our product candidates.***

Glybera or our product candidates may prove to have undesirable or unintended side effects, toxicities or other characteristics that may preclude our obtaining additional marketing approval or prevent or limit commercial use. In our clinical development program for Glybera, there were a total of 48 serious adverse events, two of which were determined to be related to Glybera, a pulmonary embolism and fever. In our partner's clinical development program for AIP, there was one serious adverse event that was determined by the investigator not to be treatment-related. If Glybera or any of our product candidates or those of other parties are associated with undesirable side effects in clinical trials or have characteristics that are unexpected, we may need to abandon their development or limit development to more narrow uses or subpopulations in which the undesirable side effects or other characteristics are less prevalent, less severe or more acceptable from a risk-benefit perspective.

***Negative public opinion and increased regulatory scrutiny of gene therapy and genetic research may damage public perception of Glybera and our product candidates or adversely affect our ability to conduct our business or obtain further marketing approvals for Glybera and marketing approvals for our product candidates.***

Public perception may be influenced by claims that gene therapy is unsafe, and gene therapy may not gain the acceptance of the public or the medical community. In particular, our success will depend upon physicians specializing in the treatment of those diseases that our product candidates target prescribing treatments that involve the use of our product candidates in lieu of, or in addition to, existing symptomatic treatments they are already familiar with and for which greater clinical data may be available.

A generalized public backlash developed against gene therapy following the death in September 1999 of an 18-year-old who had volunteered for a gene therapy experiment at the University of Pennsylvania. Researchers at the university had infused the volunteer's liver with a gene aimed at reversing a rare metabolic disease of the liver. The procedure triggered an extreme immune-system reaction that caused multiple-organ failure in a very short time, leading to the first death to occur as a direct result of a gene therapy experiment. In addition, in two gene therapy studies in 2003, 20 subjects treated for X-linked severe combined immunodeficiency using a murine gamma-retroviral vector showed correction of the disease. However, the studies were terminated after five subjects developed leukemia.

Although none of our current product candidates utilize the gamma-retroviruses used in the 2003 studies, our product candidates do use a viral vector delivery system. The risk of cancer remains a concern for gene therapy and we cannot assure that it will not occur in any of our planned or future clinical studies. In addition, there is the potential risk of delayed adverse events following exposure to gene therapy products due to persistent biological activity of the genetic material or other components of products used to carry the genetic material.

Adverse events in our clinical trials or those conducted by other parties, even if not ultimately attributable to our product candidates, and the resulting publicity could result in increased governmental regulation, unfavorable public perception, potential regulatory delays in the testing or approval of our potential product candidates, stricter labeling requirements for those product candidates that are approved and a decrease in

demand for any such product candidates. If any such adverse events occur, commercialization of Glybera or further advancement of our clinical trials could be halted or delayed, which would have a material adverse effect on our business and operations.

### **Risks Related to the Regulatory Approval of Our Product Candidates**

***Even if we complete the necessary preclinical tests and clinical trials, we cannot predict when or if we will obtain marketing approval to commercialize a product candidate, and any approval we receive may be for a more narrow indication than we expect.***

We cannot commercialize a product until the appropriate regulatory authorities have reviewed and approved the product candidate. The development and commercialization of our product candidates, including their design, testing, manufacture, safety, efficacy, purity, recordkeeping, labeling, storage, approval, advertising, promotion, sale and distribution, are subject to comprehensive regulation by the EMA and other regulatory agencies of the member states of the European Union, by the FDA and other regulatory agencies in the United States, and similar regulatory authorities outside the European Union and the United States. Failure to obtain marketing approval for a product candidate in a specific jurisdiction will prevent us from commercializing the product candidate in that jurisdiction.

We have not received approval to market any of our products or product candidates from regulatory authorities in the United States. We received marketing authorization for Glybera from the European Commission in October 2012 under exceptional circumstances for a subset of LPLD patients, after our initial application was rejected in June 2011. We plan to file an IND with the FDA for Glybera in the first half of 2014. The FDA may not allow us to rely on the results of our prior clinical trials of Glybera, all of which were conducted outside the United States, or ultimately approve Glybera for marketing in the United States. The FDA may, for example, reject or discount the results of prior trials, or consider them to be inadequate or not well-controlled. Based on our meetings with the FDA in August and December 2013, we believe that to obtain marketing approval for Glybera in the United States, we will need to successfully conduct an adequate and appropriately controlled confirmatory clinical trial. We have not yet completed the design of this trial or prepared or submitted a protocol for this trial to the FDA. We may seek to amend the protocol for our European Union post-approval trial of Glybera so that such trial also could serve as such a confirmatory trial. The FDA may require preclinical testing or clinical trials beyond this confirmatory clinical trial as a basis for marketing approval of Glybera, which would be expensive and time consuming. If we fail to obtain marketing approval of Glybera in the United States on our anticipated timeframe, or obtain only limited approval for a specific patient population, our business could be materially adversely affected.

The process of obtaining marketing approval for our product candidates in the European Union, the United States and other countries is expensive and may take many years, if approval is obtained at all. Additional clinical trials may be required and can vary substantially based upon a variety of factors, including the type, complexity and novelty of the product candidates involved. Changes in marketing approval policies during the development period, changes in or the enactment of additional statutes or regulations, or changes in regulatory review for each submitted product application, may cause delays in the approval or rejection of an application. Regulatory authorities have substantial discretion in the approval process and may refuse to accept any application, may decide that our data are insufficient for approval, may require additional preclinical, clinical or other studies and may not complete their review in a timely manner. The FDA does not maintain a regulatory approval process similar to the EMA's marketing authorization under exceptional circumstances, which may make it more difficult to obtain marketing authorization for Glybera or other product candidates in the United States. Further, any marketing approval we ultimately obtain may be for only limited indications, or be subject to stringent labeling or other restrictions or post-approval commitments that render the approved product not commercially viable. For example, we received marketing authorization for Glybera in the European Union only for a restricted patient population and other regulatory agencies may approve Glybera only with a similarly limited label, which limits our addressable



market. Further, Glybera received marketing approval subject to post-approval restrictions including the requirement to conduct a post-approval clinical study, and if we fail to adequately satisfy these post-approval requirements the EMA may withdraw its approval.

If we experience delays in obtaining approval or if we fail to maintain approval of Glybera in the European Union or obtain approval of Glybera in the United States or elsewhere or of any of our product candidates in the United States or other countries, the commercial prospects for Glybera or our other product candidates may be harmed and our ability to generate revenues will be materially impaired.

***The European Commission authorized marketing of Glybera under exceptional circumstances, and only after its subsidiary committees had made negative decisions involving the use of Glybera for the treatment of all patients with LPLD.***

The process for obtaining approval of Glybera in the European Union was protracted and complicated by initial decisions against approval by the committees charged with review of our marketing authorization application. In their initial decision in June 2011, both the CAT and the Committee for Human Medicinal Products, or CHMP, determined that the benefit-risk balance for Glybera was negative for the treatment of all patients with LPLD.

In June 2012, the CAT gave a positive opinion and the CHMP then reassessed Glybera and recommended approval for adult patients diagnosed with familial LPLD and suffering from severe or multiple pancreatitis attacks despite dietary fat restrictions. This was a more restricted patient population than we had sought in our original application. The European Commission granted this approval in October 2012, subject to certain conditions including additional post-marketing studies for efficacy.

Our receipt of marketing authorization under exceptional circumstances in the European Union does not provide any assurance that we will be able to obtain marketing authorization for Glybera elsewhere, including in the United States, or for our other gene therapies in any country.

***Our product, Glybera, was approved in Europe through a special regulatory scheme allowing for marketing of certain products under exceptional circumstances. A similar pathway to approval does not exist in the United States. As a result, approval of Glybera in Europe does not guarantee or increase the likelihood of approval of Glybera by the FDA in the United States.***

In October 2012, the European Commission authorized marketing of Glybera under exceptional circumstances. The FDA does not maintain a regulatory approval process similar to the EMA's marketing authorization under exceptional circumstances, which may make it more difficult to obtain approval for Glybera in the United States. In the United States, the FDA will generally only approve a product on the basis of two full clinical studies that contain substantial evidence of the safety and efficacy of the proposed new product. Clinical trials in the United States must be conducted in accordance with GCP requirements. A protocol for each clinical trial and any subsequent protocol amendments must be submitted to the FDA as part of an IND. Each institution participating in the clinical trial is subject to the initial approval, continuing review, and at least annual reapproval, by an IRB.

Given the differences between the regulatory schemes for approval of new products in Europe and the United States, approval of Glybera in the European Union does not assure or increase the likelihood of approval of the product in the United States. In addition, the FDA may not allow us to rely on the results of our prior clinical trials of Glybera, all of which were conducted outside the United States. The FDA may, for example, reject or discount the results of prior trials, or consider them to be inadequate or not well-controlled. In addition, the FDA may not conclude that the results of the trials demonstrate that Glybera is safe or effective, or may otherwise require additional clinical trials as a basis for marketing approval, which would be expensive and time consuming. If we fail to obtain marketing approval in the United States on our anticipated timeframe, or obtain only limited approval for a specific patient population, our business could be materially adversely affected.



***The FDA will require us to conduct comparability studies evaluating the products manufactured at our Amsterdam facility with those to be manufactured at our Lexington, Massachusetts facility, which is currently under construction. Those studies and their results could substantially delay or preclude our ability to commercialize Glybera and our product candidates in the United States.***

The FDA maintains strict requirements governing the manufacturing process for biologics. When a manufacturer seeks to modify or change that process, the FDA typically requires the applicant to conduct non clinical and, depending on the magnitude of the changes, potentially clinical comparability studies that evaluate the potential differences in the product resulting from the change in the manufacturing process. In connection with any application we may file with the FDA seeking marketing approval for Glybera or any of our other product candidates in the United States, we will be required to conduct comparability studies assessing product manufactured at our facility in Amsterdam with product to be manufactured at our facility in Lexington, Massachusetts, which we are currently building out and equipping. The FDA may be especially concerned about the need for such a comparability study for Glybera if the clinical studies on which we rely for approval of our application only involved product manufactured at our facility in the Netherlands and if we intend to market only product manufactured in Lexington in the United States.

Delays in designing and completing a comparability study to the satisfaction of FDA could delay or preclude our development and commercialization plans and, thereby, limit our revenues and growth. For example, for Glybera, we may attempt to show comparability of the product manufactured at the different facilities through the use of non-clinical data, such as potency assays and animal studies. There is a risk that such data may not show acceptable comparability of the product manufactured at the different sites to the satisfaction of FDA. In addition, in the event that the FDA does not accept such non-clinical comparability data, we may need to conduct a study involving dosing of patients with product from our Lexington facility. That study may result in a delay of the approval or launch of Glybera in the United States.

***We are subject to potentially costly post-approval requirements in the European Union that may restrict or eliminate the commercial success of Glybera.***

As part of our marketing approval under exceptional circumstances in the European Union, the EMA has approved Glybera for the treatment of a subset of adult LPLD patients with familial LPLD diagnosed by genetic testing and suffering from severe or multiple pancreatitis attacks despite dietary fat restrictions, thereby imposing significant restrictions on the indicated uses and marketing of Glybera. Further, the EMA has imposed ongoing requirements for a potentially costly post-approval study and market surveillance. Specifically, as a condition to approval we are required to complete a post-approval clinical trial and implement a disease registry for long term surveillance of patients, as well as implement risk management procedures, distribute educational materials to healthcare professionals and patients, comply with certain notification obligations and undergo annual reassessment, the outcome of which could eventually lead to a withdrawal of the approval. The expense and uncertain result of these post-approval requirements may delay, limit or terminate our commercialization plan for Glybera and adversely affect our financial position.

***The risks associated with the marketing approval process are heightened by our products' status as gene therapies.***

Glybera has been evaluated as a gene therapy by the EMA. We believe that all of our current product candidates, including Glybera, will be viewed as gene therapy products by the EMA, FDA and other regulatory authorities. Gene therapies are relatively new treatments and regulators do not have extensive experience or standard review and approval processes for gene therapies. The FDA has never approved a gene therapy product as safe and effective and, unlike the EMA, does not have an exceptional circumstances approval pathway. The EMA has approved only one gene therapy, Glybera, for a subset of LPLD patients, under exceptional circumstances, and only did so by a vote of 17 to 15 and after twice denying approval. Given the novelty and complexity of our technology, we intend to discuss with the EMA, the FDA and other regulatory authorities the appropriate scientific analysis and evaluation of our methods to

support applications for marketing approval for our product candidates. The application process will take time and resources, may require independent third-party analysis and may still not be accepted by the EMA, FDA or other regulatory authorities.

The EMA and FDA have demonstrated caution in their regulation of gene therapy treatments, and ethical and legal concerns about gene therapy and genetic testing may result in additional regulations or restrictions on the development and commercialization of our product candidates that are difficult to predict. For example, the EMA advocates a risk-based approach to the development of a gene therapy product. Agencies at both the United States federal and state level, as well as congressional committees and foreign governments, have sometimes expressed interest in further regulating biotechnology. Such action may delay or prevent commercialization of some or all of our product candidates. For example, in 2003, the FDA suspended 27 gene therapy trials involving several hundred patients after learning that a child treated in France had developed a condition resembling leukemia. Although the FDA was not aware that any of the patients treated in the 27 American trials had suffered illnesses similar to that of the infant in France, it nevertheless took precautions. This temporary halt, the largest such action involving gene therapy trials, was a setback for the field.

Regulatory requirements affecting gene therapy have changed frequently and may continue to change. For example, the European Commission conducted a public consultation in early 2013 on the application of European Union legislation that governs advanced therapy medicinal products, including gene therapy products, that could result in changes in the data we need to submit to the EMA in order for our product candidates to gain regulatory approval. In addition, divergent scientific opinions among the various bodies involved in the review process may result in delays and require additional resources and may ultimately result in rejection. For further discussion about the regulation we face in Europe and the United States, please see "Business—Government Regulation and Reimbursement."

In the United States, the FDA has established the Office of Cellular, Tissue and Gene Therapies within the Agency's Center for Biologics Evaluation and Research, or CBER, to consolidate the review of gene therapy and related products, and the Cellular, Tissue and Gene Therapies Advisory Committee to advise CBER on its review. Gene therapy clinical trials conducted at institutions that receive funding for recombinant DNA research from the NIH are also subject to review by the NIH Office of Biotechnology Activities' Recombinant DNA Advisory Committee, or the RAC. Although the FDA decides whether individual protocols may proceed, the RAC review process can impede the initiation of a clinical trial, even if the FDA has reviewed the study and approved its initiation. In addition, the FDA can put an IND on clinical hold even if the RAC has provided a favorable review. Also, before a clinical trial can begin at an NIH-funded institution, that institution's institutional review board, or IRB, and its Institutional Biosafety Committee will review the proposed clinical trial to assess the safety of the study. Adverse developments in clinical trials of gene therapy products conducted by others may cause the FDA or other regulatory bodies to change the requirements for approval of any of our product candidates.

The FDA and the EMA have issued various guidance documents pertaining to gene therapy products, with which we likely must comply to gain regulatory approval of any of our product candidates in the United States or European Union, respectively. Those guidance documents may require us, for example, to observe the subjects of our clinical trials for a longer period than normal following completion of a trial, or to undertake more extensive preclinical assessments, in particular concerning our vector and transgene expression cassette system, before beginning clinical trials at all. We also may need to conduct environmental risk assessments and special long term studies to monitor the safety and efficacy of our products. The close regulatory scrutiny of gene therapy products may result in delays and increased costs, and may ultimately lead to any gene therapy product not being approved.

These regulatory review committees and advisory groups and the new guidelines they promulgate may lengthen the regulatory review process, require us to perform additional studies, increase our development costs, lead to changes in regulatory positions and interpretations, delay or prevent approval and

commercialization of these product candidates or lead to significant post-approval limitations or restrictions. As we advance our product candidates, we will be required to consult with these regulatory and advisory groups, and comply with applicable guidelines. If we fail to do so, we may be required to delay or discontinue development of our product candidates. Delay or failure to obtain, or unexpected costs in obtaining, the regulatory approval necessary to bring a potential product to market could decrease our ability to generate sufficient product revenues to maintain our business.

***If we are not able to obtain or maintain orphan product exclusivity for any of our product candidates for which we seek this status, or if our competitors are able to obtain orphan product exclusivity before we do, we may not be able to obtain approval for our competing products for a significant period of time.***

Regulatory authorities in some jurisdictions, including the European Union and the United States, may designate drugs for relatively small patient populations as orphan drugs. Under the Orphan Drug Act, the FDA may designate a product as an orphan drug if it is a drug intended to treat a rare disease or condition, which is generally defined as a patient population of fewer than 200,000 individuals annually in the United States or not more than five in 10,000 people in the European Union. Generally, if a product with an orphan drug designation subsequently receives the first marketing approval for the indication for which it has such designation, the product is entitled to a period of market exclusivity, which precludes the EMA or FDA from approving another marketing application for the same drug for the same indication for that time period. The applicable period is seven years in the United States and ten years in the European Union following marketing approval. The European Union exclusivity period can be reduced to six years if a drug no longer meets the criteria for orphan drug designation, for example if the drug is sufficiently profitable so that market exclusivity is no longer justified.

Orphan drug exclusivity may be lost if the EMA or FDA determines that the request for designation was materially defective, or if the manufacturer is unable to assure sufficient quantity of the drug to meet the needs of patients with the rare disease or condition or if the incidence and prevalence of patients who are eligible to receive the drug in these markets materially increase. Although we have obtained orphan designation for Glybera in the European Union and the United States, even after an orphan drug is approved, the same drug can subsequently be approved for the same condition if the competent regulatory agency concludes that the later drug is clinically superior in that it is shown to be safer, more effective or makes a major contribution to patient care.

If we lose orphan drug exclusivity or if our competitors obtain orphan drug exclusivity before we do, we may be precluded from obtaining marketing authorization or we may lose out on the potential benefits of market exclusivity.

***Fast track designation by the FDA may not lead to a faster development or regulatory review or approval process, and it does not increase the likelihood that any of our product candidates will receive marketing approval in the United States.***

We may in the future seek fast track designation for Glybera or other product candidates as appropriate in the United States. If a drug is intended for the treatment of a serious or life-threatening condition or disease and the drug demonstrates the potential to address unmet medical needs for this condition, the drug sponsor may apply for FDA fast track designation. The FDA has broad discretion whether or not to grant this designation, so even if we believe a particular product candidate is eligible for this designation, we cannot assure you that the FDA would decide to grant it. Even if we do apply for and receive fast track designation, we may not experience a faster development, review or approval process compared to conventional FDA procedures. The FDA may withdraw fast track designation if it believes that the designation is no longer supported by data from our clinical development program.

***Breakthrough therapy designation by the FDA may not lead to a faster development or regulatory review or approval process, and it does not increase the likelihood that any of our product candidates will receive marketing approval in the United States.***

We may in the future apply for breakthrough therapy designation for Glybera or other product candidates in the United States. A breakthrough therapy is defined as a product that is intended, alone or in combination with one or more other drugs, to treat a serious or life-threatening disease or condition, and for which preliminary clinical evidence indicates substantial improvement over existing therapies on one or more clinically significant endpoints, such as substantial treatment effects observed early in clinical development. For drugs and biologics that have been designated as breakthrough therapies, interaction and communication between the FDA and the sponsor of the trial can help to identify the most efficient path for clinical development while minimizing the number of patients placed in ineffective control regimens. Products designated as breakthrough therapies by the FDA are also eligible for accelerated approval.

Designation as a breakthrough therapy is within the discretion of the FDA. Accordingly, even if we believe one of our products or product candidates meets the criteria for designation as a breakthrough therapy, the FDA may disagree. In any event, the receipt of a breakthrough therapy designation for a product or product candidate may not result in a faster development process, review or approval compared to products considered for approval under conventional FDA procedures and, in any event, does not assure ultimate approval by the FDA. In addition, even if one or more of our products or product candidates qualify as breakthrough therapies, the FDA may later decide that the products no longer meet the conditions for qualification or decide that the time period for FDA review or approval will not be shortened.

***Any of our product candidates for which we obtain marketing approval in the future could be subject to post-marketing restrictions or other regulatory requirements.***

Glybera and any of our product candidates for which we obtain marketing approval in the future, as well as the manufacturing process, post-approval studies and measures, labeling, advertising and promotional activities for such products, will be subject to continued requirements of and review by the FDA, EMA and other regulatory authorities. These requirements include submissions of safety and other post-marketing information and reports, registration and listing requirements, requirements relating to manufacturing, quality control, quality assurance and corresponding maintenance of records and documents and recordkeeping. Even if marketing approval of a product candidate is granted, the approval may be subject to limitations on the indicated uses for which the product may be marketed or to the conditions of approval, including the requirement to develop and implement a risk evaluation and mitigation strategy. For example, the EMA's approval of Glybera was contingent upon our agreeing to post-approval obligations described elsewhere in this prospectus.

The FDA may also impose requirements for costly post-marketing studies or clinical trials and surveillance to monitor the safety or efficacy of a product. The FDA and other agencies, including the Department of Justice, closely regulate and monitor the post-approval marketing and promotion of products to ensure that they are manufactured, marketed and distributed only for the approved indications and in accordance with the provisions of the approved label. The FDA imposes stringent restrictions on manufacturers' communications regarding off-label use and if we do not market our products for their approved indications, we may be subject to an enforcement action for off-label marketing. Violations of the Federal Food, Drug, and Cosmetic Act and other statutes, including the False Claims Act, relating to the promotion of prescription drugs may lead to investigations alleging violations of federal and state health care fraud and abuse laws, as well as state consumer protection laws. Similar regulations apply in many other foreign jurisdictions.

In addition, later discovery of previously unknown adverse events or other problems with our products, or failure to comply with regulatory requirements, may yield various adverse results, including:

- restrictions on such products or manufacturing processes;

- restrictions on the labeling or marketing of a product;
- restrictions on product distribution or use;
- requirements to conduct post-marketing studies or clinical trials;
- warning letters;
- withdrawal of the products from the market;
- refusal to approve pending applications or supplements to approved applications that we submit;
- recall of products;
- fines, restitution or disgorgement of profits or revenues;
- suspension or withdrawal of marketing approvals;
- refusal to permit the import or export of our products;
- product seizure; and
- injunctions or the imposition of civil or criminal penalties.

Non-compliance with European Union requirements regarding safety monitoring or pharmacovigilance can also result in significant financial penalties. Similarly, failure to comply with the European Union's requirements regarding the protection of personal information can also lead to significant penalties and sanctions.

Any government investigation of alleged violations of law could require us to expend significant time and resources and could generate negative publicity. The occurrence of any event or penalty may inhibit our ability or that of our collaborators to commercialize Glybera and any other products and generate revenues, and may also adversely affect our ability to obtain FDA approval. Failure to maintain marketing approval for Glybera in the European Union or to obtain regulatory approval for Glybera in other jurisdictions may also adversely affect our ability to develop other product candidates, given the general applicability of our technology platform to the development of our current and future product candidates.

#### **Risks Related to the Commercialization of Glybera and Our Product Candidates**

***If we or our collaborators are unable to commercialize Glybera or our other product candidates or experience significant delays in doing so, our business will be materially harmed.***

Our ability to generate product revenues will depend heavily on the successful commercialization of Glybera and development and eventual commercialization of other product candidates. The success of our product candidates will depend on several factors, including the following:

- successful completion of preclinical studies and clinical trials;
- receipt of marketing approvals from applicable regulatory authorities;
- obtaining and maintaining patent and trade secret protection and non-patent, orphan drug exclusivity for our product candidates;
- completing the build-out of, and obtaining regulatory approval for, our new manufacturing facility in Lexington, Massachusetts;
- launching commercial sales of our products, if and when approved, whether alone or in collaboration with others;
- identifying and engaging effective distributors or other third party resellers on acceptable terms in certain jurisdiction where we plan to utilize third parties for the marketing and sale of Glybera or other candidate products;
- acceptance of our products, if and when approved, by patients, the medical community and third party payors;
- effectively competing with other therapies;
- obtaining and maintaining healthcare coverage and adequate reimbursement; and

- complying with post-approval requirements of the EMA and maintaining a continued acceptable overall safety profile based on the EMA's risk-benefit analysis.

Failure to achieve or implement any of these elements could result in significant delays or an inability to successfully commercialize Glybera or our product candidates, which could materially harm our business.

***The affected populations for Glybera and product candidates may be smaller than we or third parties currently project, which may affect the addressable markets for Glybera and product candidates.***

Our projections of the number of people who have the diseases we are seeking to treat, as well as the subset of people with these diseases who have the potential to benefit from treatment with Glybera or our product candidates, are estimates based on our knowledge and understanding of these diseases. The total addressable market opportunity for Glybera and our product candidates will ultimately depend upon a number of factors including the diagnosis and treatment criteria included in the final label, if approved for sale in specified indications, acceptance by the medical community, patient access and product pricing and reimbursement. Prevalence estimates are frequently based on information and assumptions that are not exact and may not be appropriate, and the methodology is forward-looking and speculative. The process we have used in developing an estimated prevalence range for the indications we are targeting has involved collating limited data from multiple sources. Accordingly, the prevalence estimates included in this prospectus should be viewed with caution. Further, the data and statistical information used in this prospectus, including estimates derived from them, may differ from information and estimates made by our competitors or from current or future studies conducted by independent sources.

The use of such data involves risks and uncertainties and is subject to change based on various factors. Our estimates may prove to be incorrect and new studies may change the estimated incidence or prevalence of the diseases we seek to address. The number of patients with the diseases we are targeting in the European Union, the United States and elsewhere may turn out to be lower than expected or may not be otherwise amenable to treatment with our products, or new patients may become increasingly difficult to identify or access, all of which would adversely affect our results of operations and our business.

***Glybera, and any other product candidate that receives marketing approval in the future, may fail to achieve the degree of market acceptance by physicians, patients, third-party payors and others in the medical community necessary for commercial success.***

Doctors may be reluctant to accept a gene therapy as a treatment option or, where available, choose to continue to rely on existing symptomatic treatments. If Glybera does not achieve an adequate level of acceptance, we may not generate significant revenues from this product and we may never achieve profitability. The degree of market acceptance of Glybera, as well as of any of our product candidates that receive marketing approval in the future, will depend on a number of factors, including:

- the efficacy and potential advantages of our therapies compared with alternative treatments;
- our ability to convince payors of the long-term cost-effectiveness of our therapies and, consequently, the availability of third-party coverage and adequate reimbursement;
- the limitations on use and label requirements imposed by regulators;
- the convenience and ease of administration of our gene therapies, which in the case of Glybera requires spinal anaesthesia and multiple intramuscular injections, compared to alternative treatments;
- the willingness of the target patient population to try new therapies, especially a gene therapy, and of physicians to administer these therapies;
- the strength of marketing and distribution support;
- the prevalence and severity of any side effects; and
- any restrictions on the use of our products.

In the case of Glybera in the European Union, we are required to put in place a restricted access program to ensure that the product is used appropriately when the diagnosis is confirmed, mandating that the product only be supplied to doctors who have received the appropriate educational materials and only be used to treat patients participating in a registry to monitor the outcome of patients treated with Glybera. These requirements may further limit our ability to gain sufficient market acceptance.

***If our collaboration with Chiesi is not successful, we may not effectively commercialize Glybera in the European Union and other countries covered by our partnership with Chiesi.***

We have entered into a collaboration with Chiesi for the commercialization of Glybera in the European Union, China, Russia and other specified countries. As a result, we are dependent on the efforts of Chiesi to successfully commercialize Glybera in these countries. There is a risk that Chiesi:

- may not perform its obligations as expected;
- may have difficulties gaining acceptance of the use of Glybera in the clinical community and achieving satisfactory pricing and reimbursement of Glybera;
- may terminate, or may elect not to continue or renew, our commercialization arrangements based on changes in its strategic focus or available funding, or external factors, such as an acquisition, that divert resources or create competing priorities; and
- may not commit sufficient resources to the marketing and distribution of Glybera.

In addition, we are required to manufacture Glybera for sale by Chiesi. Should we encounter manufacturing problems, we may fail to adequately supply Glybera to Chiesi. If any of these circumstances related to our collaboration with Chiesi are realized, they may adversely affect the commercial success of Glybera in the European Union and other countries covered by our partnership with Chiesi.

***We face substantial competition, which may result in others discovering, developing or commercializing products before or more successfully than we do.***

The development and commercialization of new biotechnology and biopharmaceutical products, including gene therapies, is highly competitive. We may face competition with respect to Glybera and our current product candidates, as well as with respect to any product candidates that we may seek to develop or commercialize in the future, from large and specialty pharmaceutical companies and biotechnology companies worldwide, who currently market and sell products or are pursuing the development of products for the treatment of many of the disease indications for which we are developing our product candidates. Potential competitors also include academic institutions, government agencies and other public and private research organizations that conduct research, seek patent protection and establish collaborative arrangements for research, development, manufacturing and commercialization. In recent years, there has been a significant increase in commercial and scientific interest and financial investment in gene therapy as a therapeutic approach, which has intensified the competition in this area.

We are aware of several companies focused on developing gene therapies in various indications, including AGTC, Asklepios, Audentes Therapeutics, BioMarin, bluebird bio, Dimension/Regen X, Oxford BioSciences, Sangamo BioScience, and Spark Therapeutics, as well as several companies addressing other methods for modifying genes and regulating gene expression. We may also face competition with respect to the treatment of some of the diseases that we are seeking to target with our gene therapies from protein pharmaceuticals under development at pharmaceutical and biotechnology companies, including Pfizer, Baxter, Bayer, Novo Nordisk, Genzyme, Shire, BioMarin and Biogen Idec. We must also compete with existing standards of care, therapies and symptomatic treatments, as well as any new therapies that may become available in the future for the indications we are targeting.

Our commercial opportunity could be reduced or eliminated if our competitors develop and commercialize products that are safer, more effective, have fewer or less severe side effects, are more convenient or are less expensive than the products that we develop. Our competitors also may obtain EMA, FDA or other



regulatory approval for their products more rapidly than we do, which could result in our competitors establishing a strong market position before we are able to enter the market. Because we expect that gene therapy patients may generally require only a single administration, we believe that the first gene therapy product to enter the market for a particular indication will likely enjoy a significant commercial advantage, and may also obtain market exclusivity under applicable orphan drug regimes.

Many of the companies with which we are competing or may compete in the future have significantly greater financial resources and expertise than we do in research and development, manufacturing, preclinical testing, conducting clinical trials, obtaining regulatory approvals and marketing approved products. Mergers and acquisitions in the pharmaceutical and biotechnology industries may result in more resources being concentrated among a smaller number of our competitors. Smaller and other early-stage companies may also prove to be significant competitors, particularly through collaborative arrangements with large and established companies. These third parties compete with us in recruiting and retaining qualified scientific and management personnel, establishing clinical trial sites and patient registration for clinical trials, as well as in acquiring technologies complementary to, or necessary for, our programs.

Even if we are successful in achieving regulatory approval to commercialize a product candidate faster than our competitors, it is conceivable that we may face competition from biosimilars. In the United States, the Biologics Price Competition and Innovation Act of 2009 authorized the FDA to approve products that are "highly similar," or biosimilar, to or "interchangeable" with an FDA-approved biological product following expiration of a 12 year period of exclusivity. In the European Union, a competitor may reference data from biological products already approved, but will not be able to get on the market until ten years after the time of approval, although that may be extended to 11 years under specified circumstances. If competitors are able to obtain marketing approval for biosimilars after the applicable period of non-patent exclusivity expires, the attendant competitive pressure and consequences. Expiration or successful challenge of our applicable patent rights could also trigger competition from other products.

### **Risks Related to Our Dependence on Third Parties for Glybera and our Product Pipeline**

***We rely on third parties for important aspects of our development programs. If these parties do not perform successfully or if we are unable to maintain any of our collaboration arrangements, our business could be adversely affected.***

We have entered into collaborations with other companies and academic research institutions with respect to important elements of our commercial and development programs. For example, we have collaboration agreements with Chiesi, for both commercialization of Glybera in the European Union and certain other countries and co-development and commercialization of our hemophilia B program, and development programs with Digna Biotech, Institut Pasteur and UCSF. We believe that these arrangements provide us with access to important technologies and capabilities, and in many cases to data from our collaborators' preclinical and clinical development programs. Our collaboration with Chiesi has also provided us with important funding for our Glybera and hemophilia B development programs.

Our existing collaborations, and any future collaborations we enter into, may pose a number of risks, including the following:

- collaborators have significant discretion in determining the efforts and resources that they will apply to these collaborations;
- in our current collaborations, we generally have limited or no control over the design or conduct of clinical trials sponsored by our collaborators;
- if our collaborators do not conduct the clinical trials they sponsor in accordance with regulatory requirements or stated protocols, we will not be able to rely on the data produced in such trials in our further development efforts;



- collaborators may not perform their obligations as expected;
- collaborators may also have relationships with other entities, some of which may be our competitors;
- collaborators may not pursue development and commercialization of any product candidates or may elect not to continue or renew development or commercialization programs based on clinical trial results, changes in the collaborators' strategic focus or available funding, or external factors, such as an acquisition, that divert resources or create competing priorities;
- collaborators may delay clinical trials, provide insufficient funding for a clinical trial program, stop a clinical trial or abandon a product candidate, repeat or conduct new clinical trials or require a new formulation of a product candidate for clinical testing;
- collaborators could independently develop, or develop with third parties, products that compete directly or indirectly with our products or product candidates if the collaborators believe that competitive products are more likely to be successfully developed or can be commercialized under terms that are more economically attractive than ours;
- our collaboration arrangements may impose restrictions on our ability to undertake other development efforts that may appear to be attractive to us;
- product candidates discovered in collaboration with us may be viewed by our collaborators as competitive with their own product candidates or products, which may cause collaborators to cease to devote resources to the commercialization of our product candidates;
- a collaborator with marketing and distribution rights to Glybera or one or more of our product candidates that achieve regulatory approval may not commit sufficient resources to the marketing and distribution of such product or products;
- disagreements with collaborators, including disagreements over proprietary rights, contract interpretation or the preferred course of development, could cause delays or termination of the research, development or commercialization of product candidates, lead to additional responsibilities for us, or result in litigation or arbitration, any of which would be time-consuming and expensive;
- collaborators may not properly maintain or defend our intellectual property rights or may use our proprietary information in such a way as to invite litigation that could jeopardize or invalidate our intellectual property or proprietary information or expose us to potential litigation;
- collaborators may infringe the intellectual property rights of third parties, which may expose us to litigation and potential liability; and
- collaborations may in some cases be terminated for the convenience of the collaborator and, if terminated, we could be required to expend additional funds to pursue further development or commercialization of the applicable product or product candidates.

If our collaborations do not result in the successful development and commercialization of products or if one of our collaborators terminates its agreement with us, we may not receive any future research funding or milestone or royalty payments under the collaboration. If we do not receive the funding we expect under these agreements, our development of our product candidates could be delayed and we may need additional resources to develop our product candidates. All of the risks relating to product development, regulatory approval and commercialization described in this prospectus also apply to the activities of our development collaborators.

***If we are unable to enter into additional collaborations in the future, or if our new collaborations are not successful, we may not be able to develop or market our product candidates or obtain a strategic position in the development of new gene therapies.***

We believe collaborations enable us to gain access to early-stage clinical programs and related data, as well as to promising transgenes and other intellectual property, with limited financial investment by us. Part of our strategy is to leverage our experience and expertise in gene therapy research and development, as well as our proprietary manufacturing capabilities, to be an attractive collaborator for academic research institutions and biotechnology and pharmaceutical companies seeking to advance their programs into larger, late-stage clinical trials that require commercial-scale manufacturing. We face significant competition from other gene therapy, biotechnology and pharmaceutical companies, and we may be unable to attract suitable collaborators or reach agreements with them on acceptable terms, which could limit our access to attractive development programs.

For some of our product candidates, particularly for chronic and degenerative diseases that will require large clinical trials and a retail sales force to address the market, we may in the future collaborate with pharmaceutical and biotechnology companies for development and potential commercialization. We face significant competition in seeking appropriate collaborators. Our ability to reach a definitive agreement for a collaboration will depend, among other things, upon our assessment of the collaborator's resources and expertise, the terms and conditions of the proposed collaboration and the proposed collaborator's evaluation of a number of factors. Many of our agreements with our licensors, including our agreements with the NIH, require us to obtain consent from the licensor before we can enter into arrangements involving the sublicensing of technology we have licensed from such licensors. Our licensors may withhold such consent, or may provide such consent only if we agree to reduce our rights or increase our financial or other obligations to them. Obtaining such consent may also hamper our ability to enter into collaboration arrangements on a timely basis.

If we are unable to reach agreements with suitable collaborators on a timely basis, on acceptable terms, or at all, we may have to curtail the development of a product candidate, reduce or delay its development or one or more of our other development programs, delay its potential commercialization or reduce the scope of any sales or marketing activities, or increase our expenditures and undertake development or commercialization activities at our own expense. If we elect to fund and undertake development or commercialization activities on our own, we may need to obtain additional expertise and additional capital, which may not be available to us on acceptable terms or at all. If we fail to enter into collaborations and do not have sufficient funds or expertise to undertake the necessary development and commercialization activities, we may not be able to further develop our product candidates or bring them to market or continue to develop our product platform, and our business may be materially and adversely affected.

We do not currently have a sales or marketing infrastructure and have no experience in the sale, marketing or distribution of pharmaceutical products. To achieve commercial success for Glybera in the European Union and certain other countries, we are dependent on Chiesi's sales and marketing infrastructure. We may enter into similar arrangements with other parties in respect of the commercialization of products in other jurisdictions. Entering into arrangements with other third parties to perform these services may result in lower product revenues and profitability, if any, than if we were to market, sell and distribute Glybera or other products ourselves. In addition, we may not be successful in entering into arrangements with third parties in the future to sell, market and distribute our product candidates, including Glybera in other territories, or may be unable to do so on terms that are favorable to us. We likely will have little control over such third parties, and any of them may fail to devote the necessary resources and attention to sell and market our products effectively.

## Risks Related to Our Manufacturing

***Gene therapies, including Glybera, are complex and difficult to manufacture. We could experience production problems that result in delays in our development or commercialization schedules or otherwise adversely affect our business.***

We manufacture Glybera and clinical supplies of our product candidates ourselves in our facility in Amsterdam and plan to commence production in our Lexington, Massachusetts facility, which is currently under construction. The insect-cell based manufacturing process we use to produce Glybera and our other product candidates is highly complex and in the normal course is subject to production difficulties. A number of factors could cause production interruptions, including equipment malfunctions, facility contamination, labor problems, raw material shortages or contamination, natural disasters, disruption in utility services, terrorist activities, human error or disruptions in the operations of our suppliers.

Our viral vectors require processing steps that are more complex than those required for most chemical pharmaceuticals. Moreover, unlike chemical pharmaceuticals, the physical and chemical properties of a biologic such as ours generally cannot be adequately characterized prior to manufacturing the final product. As a result, an assay of the finished product is not sufficient to ensure that the product will perform in the intended manner. Accordingly, we employ multiple steps to attempt to control our manufacturing process to assure that the process works and the product or product candidate is made strictly and consistently in compliance with the process. Problems with the manufacturing process, even minor deviations from the normal process, could result in product defects or manufacturing failures that result in lot failures, product recalls, product liability claims and insufficient inventory. We may encounter problems achieving adequate or clinical-grade materials that meet EMA, FDA or other applicable standards or specifications with consistent and acceptable production yields and costs.

In addition, the EMA, FDA and other regulatory bodies may require us to submit samples of any lot of any approved product together with the protocols showing the results of applicable tests at any time. Under some circumstances, the EMA, FDA or other regulatory bodies may require that we not distribute a lot until the agency authorizes its release. Slight deviations anywhere in the manufacturing process, including stability and quality control, may result in unacceptable changes in the products that could result in lot failures or product recalls. Lot failures or product recalls could cause us to delay product launches or clinical trials, which could be costly to us and otherwise harm our business.

We also may encounter problems hiring and retaining the experienced specialist personnel needed to operating our manufacturing process, which could result in delays in our production or difficulties in maintaining compliance with applicable regulatory requirements.

Any problems in our manufacturing process or facilities could make us a less attractive collaborator for academic research institutions and other parties, which could limit our access to additional attractive development programs.

***Any contamination in our manufacturing process, shortages of raw materials or failure of any of our key suppliers to deliver necessary components of our platform could result in delays in our clinical development or marketing schedules.***

Given the nature of biologics manufacturing, there is a risk of contamination. Any contamination could materially adversely affect our ability to produce our gene therapies, including Glybera, on schedule and could therefore harm our results of operations and cause reputational damage.

Some of the raw materials required in our manufacturing process are derived from biological sources. Such raw materials are difficult to procure and may also be subject to contamination or recall. A material shortage, contamination, recall, or restriction on the use of biologically derived substances in the manufacture of Glybera or our product candidates could adversely impact or disrupt the commercial

manufacturing of Glybera or the production of clinical material, which could materially and adversely affect our operating results and development timelines.

We rely on single suppliers for the supply and manufacture of certain components of our technology. Specifically, we have only one source of supply for some of the materials used in the chromatography step of our manufacturing process. We are not currently seeking to establish secondary suppliers for these materials. We may not be successful in establishing secondary suppliers on acceptable terms, if at all, should our suppliers discontinue supply of these materials. Further, these suppliers are not required to give us advance notice in the event they discontinue supply of the relevant materials. Should our ability to procure these material components from our sole suppliers be compromised, our ability to continuously operate would be impaired until an alternative supplier is sourced, qualified and tested, which could limit our ability to produce a commercial supply of Glybera, delay the development programs of Glybera and our other product candidates and harm our business.

***Delays in completing and receiving regulatory approvals for our new U.S. manufacturing facility could delay our development and commercialization plans and thereby limit our revenues and growth.***

We are expending significant funds for the build-out of our leased 53,000 square foot manufacturing facility in Lexington, Massachusetts. This project may result in unanticipated delays and cost more than expected due to a number of factors, including regulatory requirements. If construction or regulatory approval of our new facility is delayed, we may not be able to manufacture sufficient quantities of Glybera or our product candidates, which would limit our commercialization and development activities and our opportunities for growth. Cost overruns associated with this facility could also require us to raise additional funds from external sources, which may be unavailable on favorable terms or at all.

***Our manufacturing facility in Amsterdam is, and our facility in Lexington that is under construction will be, subject to significant government regulations and approvals, which are often costly. If we fail to comply with these regulations or maintain these approvals, our business will be materially harmed.***

Our manufacturing facility in Amsterdam is, and our new facility in Lexington will be, subject to ongoing regulation and periodic inspection by the EMA, FDA and other regulatory bodies to ensure compliance with cGMP. Any failure to follow and document our adherence to such cGMP regulations or other regulatory requirements may lead to significant delays in the availability of products for commercial use or clinical study, may result in the termination of or a hold on a clinical study, or may delay or prevent filing or approval of marketing applications for our products. In addition, if we alter our manufacturing process for gene therapies, including Glybera, or during the development of a product candidate, the EMA, FDA or other regulatory authorities may require additional testing and clinical studies to ensure adequate safety and efficacy.

To monitor our compliance with applicable regulations, the EMA, FDA and comparable agencies in other jurisdictions routinely conduct inspections of our facilities and may identify potential deficiencies for us to address. For example, the FDA issues what are referred to as "FDA Form 483s" that set forth observations and concerns that are identified during its inspections. We may receive Form 483s in the future. Failure to satisfactorily address the concerns or potential deficiencies identified in a Form 483 could result in us being issued a warning letter, a notice of what the FDA believes to be significant regulatory violations requiring prompt corrective actions. If we fail to adequately respond to a warning letter, or otherwise fail to comply with applicable regulatory requirements, we could be subject to enforcement, remedial and/or punitive actions by the FDA or other regulatory authorities.

Failure to comply with applicable regulations could also result in the EMA, FDA or other applicable authorities taking various actions, including:

- levying fines and other civil penalties;
- imposing consent decrees or injunctions;

- a requirement to suspend or put on hold one or more of our clinical trials;
- suspending or withdrawing regulatory approvals;
- delaying or refusing to approve pending applications or supplements to approved applications;
- suspending manufacturing activities or product sales, imports or exports;
- requiring us to communicate with physicians and other customers about concerns related to actual or potential safety, efficacy, and other issues involving our products;
- mandating product recalls or seizing products;
- imposing operating restrictions; and
- seeking criminal prosecutions.

Any of the foregoing could materially harm our business.

***Our use of viruses, chemicals and other hazardous materials requires us to comply with regulatory requirements and exposes us to significant potential liabilities.***

Our development and manufacturing processes involve the use of viruses, chemicals and other hazardous materials, and produce waste products. Accordingly, we will be subject to federal, state and local laws and regulations in the United States, and are subject to comparable regulations in the Netherlands, governing the use, manufacture, distribution, storage, handling, treatment and disposal of these materials. In addition to ensuring the safe handling of these materials, applicable requirements require increased safeguards and security measures for many of these agents, including controlling access and screening of entities and personnel who have access to them, and establishing a comprehensive national database of registered entities. In the event of an accident or failure to comply with environmental, occupational health and safety and export control laws and regulations, we could be held liable for damages that result, and any such liability could exceed our assets and resources.

#### **Risks Related to Our Intellectual Property**

***We license intellectual property from third parties, and such licenses may not provide adequate rights, may not be available in the future on commercially reasonable terms or at all, or our licensors may be unable to obtain and maintain patent protection for the technology or products that we license from them.***

We currently are heavily reliant upon licenses of proprietary technology from third parties that is important or necessary to the development of our technology and products, including technology related to our manufacturing process, our vector platform, our gene cassettes and the therapeutic genes of interest we are using. These and other licenses may not provide adequate rights to use such technology in all relevant fields of use. Licenses to additional third-party technology that may be required for our development programs may not be available in the future or may not be available on commercially reasonable terms, which could have a material adverse effect on our business and financial condition.

In some circumstances, we may not have the right to control the preparation, filing and prosecution of patent applications, or to maintain the patents, covering technology that we license from third parties. In addition, some of our agreements with our licensors require us to obtain consent from the licensor before we can enforce patent rights, and our licensor may withhold such consent or may not provide it on a timely basis. Therefore, we cannot be certain that these patents and applications will be prosecuted and enforced in a manner consistent with the best interests of our business. In addition, if third parties who license patents to us fail to maintain such patents, or lose rights to those patents, the rights we have licensed may be reduced or eliminated.

***Our intellectual property licenses with third parties may be subject to disagreements over contract interpretation, which could narrow the scope of our rights to the relevant intellectual property or technology or increase our financial or other obligations to our licensors.***

The agreements under which we currently license intellectual property or technology from third parties are complex, and certain provisions in such agreements may be susceptible to multiple interpretations. The resolution of any contract interpretation disagreement that may arise could narrow what we believe to be the scope of our rights to the relevant intellectual property or technology, or increase what we believe to be our financial or other obligations under the relevant agreement, either of which could have a material adverse effect on our business and financial condition. For example, we have an exclusive license from the NIH for "the development and sale of AAV5 based therapeutic products to be delivered to the brain or liver for treatment of human diseases originating in the brain or liver," other than arthritis-related diseases. We also have a non-exclusive license from the NIH for the development and sale of AAV5 based therapeutic products to treat human diseases other than those covered by our exclusive license.

We believe that our exclusive license from the NIH includes the systemic administration of AAV5-based therapeutic products so long as such therapeutic products are "to be delivered to the brain or liver for treatment of human diseases originating in the brain or liver." However, Sangamo BioSciences, Inc., or Sangamo, has announced that it has broad worldwide licenses to use AAV vectors, including AAV5 and AAV6, for research, development and commercialization of therapies for hemophilia A and B, Huntington's disease and other targets. We believe Sangamo's view may be that our exclusive license excludes systemic administration because Sangamo interprets the phrase "to be delivered to" to require direct administration into the brain or liver. Our view is that the phrase "to be delivered to" indicates the ultimate destination of the therapy and not the location where it is first introduced into the body. Although we think our interpretation is correct, there can be no assurance that a court would agree with our interpretation regarding the meaning of this phrase. If our interpretation of the phrase "to be delivered to" is incorrect, then others may obtain licenses from the NIH that may enable them to compete with us in the systemic administration of AAV5-based therapeutics for treatment of human diseases originating in the brain or liver, which could harm our business.

***If we fail to comply with our obligations in our intellectual property licenses with third parties, we could lose rights that are important to our business.***

We in-license intellectual property from third parties that is material to Glybera and all of our product candidates, including technology related to our manufacturing process, our vector platform, and the therapeutic gene cassettes we are using. Our licensing arrangements with third parties impose diligence, development and commercialization timelines, milestone payment, royalty, insurance and other obligations on us. If we fail to comply with these obligations, our counterparties may have the right to terminate these agreements, in which case we might not be able to develop, manufacture or market any product that is covered by these agreements or may face other penalties under the agreements. Such an occurrence could materially adversely affect the value of the product candidate being developed under any such agreement. Termination of these agreements or reduction or elimination of our rights under these agreements may result in our having to negotiate new or amended agreements with less favorable terms, or cause us to lose our rights under these agreements, including our rights to important intellectual property or technology.

***If we are unable to obtain and maintain patent protection for our technology and products, our ability to successfully commercialize our products may be impaired.***

We rely upon a combination of in-licensed and owned patents, trade secret protection and confidentiality agreements to protect our intellectual property. Our success depends in large part on our ability to obtain and maintain this protection in the European Union, the United States and other countries, in part by filing patent applications related to our novel technologies and product candidates. Our patents may not provide us with any meaningful commercial protection, prevent competitors from competing with us or otherwise

provide us with any competitive advantage. Our competitors may be able to circumvent our owned or licensed patents by developing similar or alternative technologies or products in a non-infringing manner.

The patent prosecution process is expensive and time-consuming, and we may not be able to file and prosecute all necessary or desirable patent applications at a reasonable cost or in a timely manner. It is also possible that we will fail to identify patentable aspects of our research and development output before it is too late to obtain patent protection.

The patent position of biotechnology and pharmaceutical companies generally is highly uncertain, involves complex legal and factual questions and has in recent years been the subject of much litigation. In addition, the laws of foreign countries may not protect our rights to the same extent as the laws of the United States. For example, European Union patent law with respect to the patentability of methods of treatment of the human body is more limited than United States law. Publications of discoveries in the scientific literature often lag behind the actual discoveries, and patent applications in the United States and other jurisdictions are typically not published until 18 months after their priority date, or in some cases at all. Therefore, we cannot know with certainty whether we were the first to make the inventions or that we were the first to file for patent protection of the inventions claimed in our owned or licensed patents or pending patent applications. As a result, the issuance, scope, validity, enforceability and commercial value of our patent rights are highly uncertain. Our pending and future patent applications may not result in patents being issued that protect our technology or products, in whole or in part, or which effectively prevent others from commercializing competitive technologies and products. Changes in either the patent laws or interpretation of the patent laws in the European Union, the United States or other countries may diminish the value of our patents or narrow the scope of our patent protection.

***If the scope of the patent protection we obtain is not sufficiently broad, our ability to successfully commercialize our technology and products may be impaired.***

The issuance of a patent is not conclusive as to its inventorship, scope, validity or enforceability, and our owned and licensed patents may be challenged in the courts or patent offices in the European Union, the United States or elsewhere. Such challenges may result in loss of exclusivity or freedom to operate or in patent claims being narrowed, invalidated or held unenforceable, in whole or in part, which could limit our ability to stop others from using or commercializing similar or identical technology and products, or limit the duration of the patent protection of our technology and products. Given the amount of time required for the development, testing and regulatory review of new product candidates, patents protecting such candidates might expire before or shortly after such candidates are commercialized. As a result, our owned and licensed patent portfolio may not provide us with sufficient rights to exclude others from commercializing products similar or identical to ours.

Recent patent reform legislation could increase the uncertainties and costs surrounding the prosecution of our patent applications and the enforcement or defense of our issued patents. In September 2011, the Leahy-Smith America Invents Act, or the Leahy-Smith Act, was signed into law. The Leahy-Smith Act includes a number of significant changes to United States patent law. These include provisions that affect the way patent applications are prosecuted and may also affect patent litigation. The United States Patent Office recently developed new regulations and procedures to govern administration of the Leahy-Smith Act, and many of the substantive changes to patent law associated with the Leahy-Smith Act and, in particular, the first to file provisions, only became effective in March 2013. Accordingly, it is not clear what, if any, impact the Leahy-Smith Act will have on the operation of our business. However, the Leahy-Smith Act and its implementation could increase the uncertainties and costs surrounding the prosecution of our patent applications and the enforcement or defense of our issued patents, all of which could have a material adverse effect on our business and financial condition.

Moreover, since September 2012, third parties now have standing to submit documents to the U.S. Patent and Trademark Office that relate to pending patent applications, known as pre-issuance submissions, for

consideration during examination of a patent application. In addition, third parties may now challenge issued patents through *inter partes* reviews and post-grant reviews which include trials before a panel of administrative patent judges. We may be subject to such pre-issuance submissions, *inter partes* reviews or post-grant reviews, or become involved in opposition, re-examination, interference or derivation proceedings challenging our patent rights or the patent rights of others. An adverse determination in any such examination, proceeding or litigation could reduce the scope of, or invalidate, our patent rights, allow third parties to commercialize our technology or products and compete directly with us, without payment to us, or result in our inability to manufacture or commercialize products without infringing third party patent rights. In addition, if the breadth or strength of protection provided by our patents and patent applications is threatened, it could dissuade companies from collaborating with us to license, develop or commercialize current or future product candidates.

***We may become involved in lawsuits to protect or enforce our patents or other intellectual property, which could be expensive, time consuming and unsuccessful.***

Competitors may infringe our owned or licensed patents or other intellectual property. To counter infringement or unauthorized use, we may be required to file infringement claims, which can be expensive and time consuming. Any claims we assert against perceived infringers could provoke these parties to assert counterclaims against us alleging that we infringe their patents. In addition, in a patent infringement proceeding, a court may decide that a patent of ours is invalid or unenforceable, in whole or in part, construe the patent's claims narrowly or refuse to stop the other party from using the technology at issue on the grounds that our patents do not cover the technology in question. An adverse result in any litigation proceeding could put one or more of our patents at risk of being invalidated or interpreted narrowly.

Even if resolved in our favor, litigation or other legal proceedings relating to intellectual property claims may cause us to incur significant expenses, and could distract our technical and management personnel from their normal responsibilities. In addition, there could be public announcements of the results of hearings, motions or other interim proceedings or developments and if securities analysts or investors perceive these results to be negative, it could have a substantial adverse effect on the price of our ordinary shares. Such litigation or proceedings could substantially increase our operating losses and reduce the resources available for development activities or any future sales, marketing or distribution activities. We may not have sufficient financial or other resources to conduct such litigation or proceedings adequately. Some of our competitors may be able to sustain the costs of such litigation or proceedings more effectively than we can because of their greater financial resources. Uncertainties resulting from the initiation and continuation of patent litigation or other proceedings could compromise our ability to compete in the marketplace. Third parties may initiate legal proceedings alleging that we are infringing their intellectual property rights, the outcome of which would be uncertain and could have a material adverse effect on the success of our business.

Our commercial success depends upon our ability, and the ability of our collaborators, to develop, manufacture, market and sell our product candidates and use our proprietary technologies without infringing the proprietary rights of third parties. There is considerable intellectual property litigation in the biotechnology and pharmaceutical industries. We may become party to, or threatened with, future adversarial proceedings or litigation regarding intellectual property rights with respect to our products and technology, including interference or derivation proceedings before the U.S. Patent and Trademark Office. Third parties may assert infringement claims against us based on existing patents or patents that may be granted in the future.



***Third parties may assert their intellectual property rights against us, which could require us to defend lawsuits, obtain licenses, and cease or delay commercializing certain product candidates.***

If we are found to infringe a third party's intellectual property rights, we could be required to obtain a license from such third party to continue developing and marketing our products and technology. However, we may not be able to obtain any required license on commercially reasonable terms or at all. Even if we were able to obtain a license, it could be non-exclusive, thereby giving our competitors access to the same technologies licensed to us. We could be forced, including by court order, to cease commercializing the infringing technology or product. In addition, we could be found liable for monetary damages, including treble damages and attorneys' fees if we are found to have wilfully infringed a patent. A finding of infringement could prevent us from commercializing our product candidates or force us to cease some of our business operations, which could materially harm our business. Claims that we have misappropriated the confidential information or trade secrets of third parties could have a similar negative impact on our business.

For example, we are aware of patents owned by third parties that relate to some aspects of our programs that are still in development. In some cases, because we have not determined the final methods of manufacture, the method of administration or the therapeutic compositions for these programs, we cannot determine whether rights under such third party patents will be needed. In addition, in some cases, we believe that the claims of these patents are invalid or not infringed, or will expire before commercialization. However, if such patents are needed and found to be valid and infringed, we could be required to obtain licenses, which might not be available on commercially reasonable terms, or to cease or delay commercializing certain product candidates, or to change our programs to avoid infringement.

***Our reliance on third parties may require us to share our trade secrets, which could increase the possibility that a competitor will discover them or that our trade secrets will be misappropriated or disclosed.***

Because we collaborate with various organizations and academic research institutions on the advancement of our gene therapy platform, we must, at times, share trade secrets with them. We seek to protect our proprietary technology in part by entering into confidentiality agreements and, if applicable, materials transfer agreements, collaborative research agreements, consulting agreements or other similar agreements with our collaborators, advisors and consultants prior to beginning research or disclosing proprietary information. These agreements typically limit the rights of the third parties to use or disclose our confidential information, such as trade secrets. Despite the contractual provisions employed when working with third parties, the need to share trade secrets and other confidential information increases the risk that such trade secrets become known by our competitors, are inadvertently incorporated into the technology of others, or are disclosed or used in violation of these agreements. Given that our proprietary position is based, in part, on our know-how and trade secrets, a competitor's discovery of our trade secrets or other unauthorized use or disclosure would impair our competitive position and may have a material adverse effect on our business.

In addition, these agreements typically restrict the ability of our collaborators, advisors and consultants to publish data potentially relating to our trade secrets. Our academic collaborators typically have rights to publish data, provided that we are notified in advance and may delay publication for a specified time in order to secure our intellectual property rights arising from the collaboration. In other cases, publication rights are controlled exclusively by us, although in some cases we may share these rights with other parties. We also conduct joint research and development programs that may require us to share trade secrets under the terms of our research and development partnerships or similar agreements.

Some courts inside and outside the United States are less willing or unwilling to protect trade secrets. If any of our trade secrets were to be lawfully obtained or independently developed by a competitor, we would have no right to prevent them, or those to whom they communicate it, from using that technology or information to compete with us.

## Risks Related to Pricing and Reimbursement

***We face uncertainty related to insurance coverage of and pricing and reimbursement for Glybera and any product candidates for which we may receive marketing approval.***

We anticipate that the cost of treatment using Glybera or our other product candidates will be significant. We expect that most patients and their families will not be capable of paying for our products themselves. There will be no commercially viable market for Glybera or our other product candidates without reimbursement from third-party payors, such as government health administration authorities, private health insurers and other organizations. Even if there is a commercially viable market, if the level of third-party reimbursement is below our expectations, our revenues and gross margins will be adversely affected and our business will be harmed.

Government authorities and other third party payors, such as private health insurers and health maintenance organizations, decide which medications they will pay for and establish reimbursement levels. Reimbursement systems vary significantly by country and by region, and reimbursement approvals must be obtained on a country-by-country basis. Government authorities and third party payors have attempted to control costs by limiting coverage and the amount of reimbursement for particular medications and procedures. Increasingly, third party payors require drug companies to provide them with predetermined discounts from list prices, are exerting influence on decisions regarding the use of particular treatments and are limiting covered indications. If coverage and adequate reimbursement are not available or reimbursement is available only at limited levels, we may not be able to successfully commercialize Glybera or any product candidate for which we obtain marketing approval.

The pricing review period and pricing negotiations for new medicines take considerable time and have uncertain results. Pricing review and negotiation often begins only after the receipt of regulatory marketing approval, and some authorities require approval of the sale price of a product before it can be marketed. In some markets, particularly the countries of the European Union, prescription pharmaceutical pricing remains subject to continuing direct governmental control and to drug reimbursement programs even after initial approval is granted, and price reductions may be imposed. Prices of medical products may also be subject to varying price control mechanisms or limitations as part of national health systems if products may be considered not to be cost-effective or where the drug company's profits are deemed excessive. In addition, pricing and reimbursement decisions in certain countries can lead to mandatory price reductions or additional reimbursement restrictions in other countries. As a result of these restrictions, Glybera, as well as any product candidates for which we may obtain marketing approval in the future, may be subject to price regulations that delay or prohibit our or our partners' commercial launch of the product. In addition, we or our collaborators may elect to reduce the price of our products in order to increase the likelihood of obtaining reimbursement approvals. In the event that countries impose prices which are not sufficient to allow us or our collaborators to generate a profit, we or our collaborators may refuse to launch the product in such countries or withdraw the product from the market. If pricing is set at unsatisfactory levels, or if the price decreases, our business could be harmed, possibly materially. If we fail to obtain and sustain an adequate level of coverage and reimbursement for our products by third party payors, our ability to market and sell our products would be adversely affected and our business would be harmed.

***Due to the generally limited addressable market for our target orphan indications and the potential for Glybera and our product candidates to offer therapeutic benefit in a single administration, we face uncertainty related to pricing and reimbursement for these product candidates.***

The relatively small market size for orphan indications and the potential for long-term therapeutic benefit from a single administration present particular challenges to pricing review and negotiation for Glybera and our product candidates for which we may obtain marketing authorization. The patient populations for Glybera and our product candidates targeted at orphan disease are relatively small. If we are unable to obtain adequate levels of reimbursement relative to the small market size in our target orphan indications,

our ability to support our development and commercial infrastructure and to successfully market and sell Glybera and other product candidates for which we may obtain marketing approval will be adversely affected.

We also anticipate that Glybera and many or all of our gene therapy product candidates may provide long-term, and potentially curative benefit with a single administration. This is a different paradigm than that of other pharmaceutical therapies, which often require an extended course of treatment or frequent administration. As a result, governments and other payors may be reluctant to provide the significant level of reimbursement that we seek at the time of administration of our gene therapies or may seek to tie reimbursement to clinical evidence of continuing therapeutic benefit over time. Although we anticipate that Glybera will need to be administered only once, there may be situations in which we may need to readminister Glybera, which may further complicate the pricing and reimbursement for Glybera. In addition, in light of the anticipated cost of these therapies, governments and other payors may be particularly restrictive in making coverage decisions. These factors could limit our commercial success and harm our business.

***Recently enacted and future legislation and related market pricing pressures may affect the coverage and pricing and reimbursement we may obtain for our products.***

A primary trend in the U.S. healthcare industry and elsewhere is cost containment. In the United States and some foreign jurisdictions, there have been a number of legislative and regulatory changes and proposed changes regarding the healthcare system that could prevent or delay marketing approval of our product candidates, restrict or regulate post-approval activities and affect our ability to profitably sell any products for which we obtain marketing approval. We expect that existing, as well as other healthcare reform measures that may be adopted in the future, may result in more rigorous coverage criteria and in additional downward pressure on the price that we receive for any approved product. Any reduction in reimbursement from government programs may result in a similar reduction in payments from private payors. The implementation of cost containment measures or other healthcare reforms may prevent us from being able to generate revenues, attain profitability, or commercialize our products.

### **Risks Related to Other Legal Compliance Matters**

***Our relationships with customers and third-party payors will be subject to applicable anti-kickback, fraud and abuse and other healthcare laws and regulations, which could expose us to criminal sanctions, civil penalties, contractual damages, reputational harm and diminished profits and future earnings.***

Healthcare providers, physicians and third-party payors will play a primary role in the recommendation and prescription of any products for which we obtain marketing approval. Our future arrangements with third-party payors and customers may expose us to broadly applicable fraud and abuse and other healthcare laws and regulations that may constrain the business or financial arrangements and relationships through which we market, sell and distribute any products for which we obtain marketing approval.

Within the European Union, the control of unlawful marketing activities is a matter of national law in each of the member states. We could face civil, criminal and administrative sanctions if any member state determines that we have breached our obligations under its national laws. Industry associations also closely monitor the activities of member companies. If these organizations or authorities name us as having breached our obligations under their regulations, rules or standards, our reputation would suffer and our business and financial condition could be adversely affected. Similarly, failure to comply with the European Union's requirements regarding the protection of personal information can also lead to significant penalties and sanctions.

If we market a product in the United States in the future, we will be subject to various federal and state laws and regulations including:

- the federal Anti-Kickback Statute, which prohibits, among other things, persons from knowingly and wilfully soliciting, offering, receiving or providing remuneration, directly or indirectly, in cash or in kind, to induce or reward, or in return for, either the referral of an individual for, or the purchase, order or recommendation of, any good or service, for which payment may be made under a federal healthcare program such as Medicare and Medicaid;
- the federal False Claims Act, which imposes criminal and civil penalties, including civil whistleblower or qui tam actions, against individuals or entities for knowingly presenting, or causing to be presented, to the federal government, claims for payment that are false or fraudulent or making a false statement to avoid, decrease or conceal an obligation to pay money to the federal government;
- the federal Health Insurance Portability and Accountability Act of 1996, or HIPAA, which imposes criminal and civil liability for executing a scheme to defraud any healthcare benefit program or making false statements relating to healthcare matters and also imposes obligations, including mandatory contractual terms, with respect to safeguarding the privacy, security and transmission of individually identifiable health information;
- federal law that requires applicable manufacturers of covered drugs to report payments and other transfers of value to physicians and teaching hospitals;
- the U.S. Foreign Corrupt Practices Act, which prohibits the offering to pay, paying or promising to pay or authorizing the payment of money or anything of value to a foreign official in order to influence any act or decision of the foreign official or secure any improper advantage in order to obtain or retain business; and
- certain state laws that require pharmaceutical companies to comply with the pharmaceutical industry's voluntary compliance guidelines and the relevant compliance guidance promulgated by the federal government and require drug manufacturers to report information related to payments and other transfers of value to physicians and other healthcare providers or marketing expenditures. State and foreign laws also govern the privacy and security of health information in some circumstances, many of which differ from each other in significant ways and often are not pre-empted by HIPAA, thus complicating compliance efforts.

We will also be subject to similar local laws in jurisdictions in which we may seek or obtain marketing authorization, or in which we may have operations or sales. Efforts to ensure that our business arrangements with third parties will comply with applicable laws and regulations will involve substantial costs. It is possible that governmental authorities will conclude that our business practices may not comply with current or future statutes, regulations or case law involving applicable fraud and abuse or other healthcare laws and regulations. If our operations, or the activities of our collaborators, distributors or other third-party agents are found to be in violation of any of these laws or any other governmental regulations that may apply to us, we may be subject to significant civil, criminal and administrative penalties, damages, fines, imprisonment, exclusion of products from government funded healthcare programs and the curtailment or restructuring of our operations. If any of the physicians or other healthcare providers or entities with whom we expect to do business is found to be not in compliance with applicable laws, they may be subject to criminal, civil or administrative sanctions, including exclusions from government funded healthcare programs.

***If we fail to comply with environmental, health and safety laws and regulations, we could become subject to fines or penalties or incur costs that could harm our business.***

We are subject to numerous environmental, health and safety laws and regulations, including those governing laboratory procedures and the handling, use, storage, treatment and disposal of hazardous materials and wastes. Our operations involve the use of hazardous and flammable materials, including chemicals and biological materials. Our operations also produce hazardous waste products. We cannot eliminate the risk of contamination or injury from these materials. In the event of contamination or injury resulting from our use of hazardous materials, we could be held liable for any resulting damages, and any liability could exceed our resources. We also could incur significant costs associated with civil or criminal fines and penalties for failure to comply with such laws and regulations.

Although we maintain employer's liability insurance to cover us for costs and expenses we may incur due to injuries to our employees resulting from the use of hazardous materials, this insurance may not provide adequate coverage against potential liabilities. We do not maintain insurance for environmental liability or toxic tort claims that may be asserted against us in connection with our storage or disposal of biological, hazardous or radioactive materials.

In addition, we may incur substantial costs in order to comply with current or future environmental, health and safety laws and regulations. These current or future laws and regulations may impair our research, development or production efforts. Our failure to comply with these laws and regulations also may result in substantial fines, penalties or other sanctions.

***Product liability lawsuits against us could cause us to incur substantial liabilities and to limit commercialization of Glybera and any products that we may develop in the future.***

We face an inherent risk of product liability related to the testing of our product candidates in human clinical trials and will face an even greater risk when we commercially sell Glybera and any other products that we may develop. If we cannot successfully defend ourselves against claims that our product candidates or products caused injuries, we will incur substantial liabilities. Regardless of merit or eventual outcome, liability claims may result in:

- decreased demand for any product candidates or products that we develop or sell;
- injury to our reputation and significant negative media attention;
- negative publicity or public opinion surrounding gene therapy;
- withdrawal of clinical trial participants;
- significant costs to defend the related litigation;
- substantial monetary awards to trial participants or patients;
- loss of revenue;
- reduced resources of our management to pursue our business strategy; and
- the inability to further develop or commercialize any products that we develop.

We currently hold €6,000,000 in clinical trial insurance coverage in the aggregate, with a per incident limit of €400,000 to €450,000, with respect to the clinical studies we conduct. Such coverage may not be adequate to cover all liabilities that we may incur. We may need to increase our insurance coverage as we expand our clinical trials and commercialize Glybera. In addition, insurance coverage is increasingly expensive. We may not be able to maintain insurance coverage at a reasonable cost or in an amount adequate to satisfy any liability that may arise.

## Risks Related to Employee Matters and Managing Growth

***Our future success depends on our ability to retain key executives and technical staff and to attract, retain and motivate qualified personnel.***

We are highly dependent on the research and development, clinical and business development expertise of our Chief Executive Officer, Jörn Aldag, our Chief Medical Officer, Christian Meyer, M.D., and our Vice President, Research and Development, Harald Petry, as well as the other principal members of our management, scientific and clinical team. Although we have entered into employment agreements with our senior management, each of them may terminate their employment on relatively short notice. We do not maintain "key person" insurance for any of our senior management or employees.

Substantially all share options outstanding under our 2012 plan will vest in full upon the closing of this offering, including those held by our senior management and key employees. Following this offering, therefore, our outstanding options will no longer have retention value, and we may not have appropriate adequate equity incentives in place to retain or motivate these key personnel.

The loss of the services of our senior management or other key employees could impede the achievement of our research, development and commercialization objectives and seriously harm our ability to successfully implement our business strategy. Furthermore, replacing senior management and key employees may be difficult and may take an extended period of time because of the limited number of individuals in our industry with the breadth and depth of skills and experience required to successfully develop, gain regulatory approval of and commercialize gene therapy products. Competition to hire from this limited pool is intense, and we may be unable to hire, train, retain or motivate these key personnel on acceptable terms.

In connection with our corporate restructuring in 2012, we lost many talented employees, including employees with an extensive understanding of our clinical programs as well as our regulatory and financial affairs. This reduction in headcount may adversely affect our ability in the future to attract and retain other qualified staff.

We also experience competition for the hiring of scientific and clinical personnel from universities and research institutions. In addition, we rely on consultants and advisors, including scientific and clinical advisors, to assist us in formulating our research and development and commercialization strategy. Our consultants and advisors may have commitments under employment, consulting or advisory contracts with other entities that may limit their availability to us. If we are unable to continue to attract and retain high quality personnel, our ability to pursue our growth strategy will be limited.

***We plan to expand our key capabilities and, as a result, may encounter difficulties in managing our growth, which could disrupt our operations. If we are unable to establish such capabilities we may not be successful in commercializing Glybera or our other product candidates in the United States or other countries, even if we receive marketing approval.***

If we receive marketing approval, we intend to build a sales, marketing and medical affairs infrastructure to market Glybera and potentially other product candidates in the United States and other countries. We currently have no experience building and training an internal sales force. We expect in the future to experience significant growth in the number of our employees and the scope of our operations, particularly in the areas of manufacturing, clinical development, regulatory affairs and sales, marketing and distribution. To manage our anticipated future growth, we will be required to implement and improve our managerial, operational and financial systems, expand our facilities and recruit and train additional qualified personnel. Recruiting and training a sales force is expensive and time-consuming and could delay any ultimate launch of Glybera or other product candidates for which we are able to obtain marketing approval in the United States and other markets. Due to our limited financial resources and the limited experience of our management team in running a company with this level of anticipated growth, we may not be able to effectively manage the expansion of our operations or recruit and train additional qualified personnel.

If the commercial launch of Glybera or any other product candidate for which we recruit a sales force and establish marketing capabilities is delayed or does not occur for any reason, we would have prematurely or unnecessarily incurred these commercialization expenses. This may be costly, and our investment would be lost if we cannot retain or reposition our sales and marketing personnel. Factors that may inhibit our efforts to commercialize Glybera or other product candidates on our own include:

- our inability to recruit, train and retain adequate numbers of effective sales, marketing and medical affairs personnel;
- the inability of sales, marketing and medical affairs personnel to obtain access to physicians or persuade adequate numbers of physicians to prescribe our gene therapies;
- the lack of complementary products to be offered by our sales personnel, which may put us at a competitive disadvantage relative to companies with more extensive product lines; and
- unforeseen costs and expenses associated with creating an independent sales and marketing organization.

If we do not successfully establish sales, marketing and medical affairs capabilities, either on our own or in collaboration with third parties, we will not be successful in commercializing Glybera or other product candidates in the United States.

### **Risks Related to this Offering and Ownership of our Ordinary Shares**

***There has been no public market for our ordinary shares prior to this offering, and you may not be able to resell our ordinary shares at or above the price you paid, or at all.***

Prior to this initial public offering, there was no established public market for our ordinary shares. Our predecessor entity, Amsterdam Molecular Therapeutics, was previously listed on EuroNext Amsterdam. Since our acquisition of the business of AMT in 2012 as part of a corporate reorganization, there has been very limited liquidity for our ordinary shares through an electronic trading platform in the Netherlands. We have applied for listing of our ordinary shares on the NASDAQ Global Market. If an active trading market for our ordinary shares does not develop after this offering, the market price and liquidity of our ordinary shares will be materially and adversely affected.

The initial public offering price for our ordinary shares will be determined by negotiations between us and the underwriters and may bear no relationship to the market price for our ordinary shares after the initial public offering. We cannot assure you that an active trading market for our ordinary shares will develop or that the market price of our ordinary shares will not decline below the initial public offering price.

***The price of our ordinary shares may be volatile and fluctuate substantially, which could result in substantial losses for purchasers of our ordinary shares in this offering.***

Our share price is likely to be volatile. The stock market in general and the market for smaller biopharmaceutical companies in particular have experienced extreme volatility that has often been unrelated to the operating performance of particular companies. As a result of this volatility, you may not be able to sell your ordinary shares at or above the initial public offering price. The market price for our ordinary shares may be influenced by many factors, including:

- the success of competitive products or technologies;
- results of clinical trials of our product candidates or those of our competitors;
- public perception of gene therapy;
- regulatory delays and greater government regulation of potential products due to adverse events;
- regulatory or legal developments in the European Union, the United States and other countries;
- developments or disputes concerning patent applications, issued patents or other proprietary rights;
- the recruitment or departure of key personnel;

- the level of expenses related to any of our product candidates or clinical development programs;
- the results of our efforts to discover, develop, acquire or in-license additional product candidates or products;
- actual or anticipated changes in estimates as to financial results, development timelines or recommendations by securities analysts;
- variations in our financial results or those of companies that are perceived to be similar to us;
- changes in the structure of healthcare payment systems;
- market conditions in the pharmaceutical and biotechnology sectors; and
- general economic, industry and market conditions.

***After this offering, our senior managers, directors and principal shareholders, if they choose to act together, will continue to have the ability to control all matters submitted to shareholders for approval.***

Upon the closing of this offering, our senior managers, and directors, combined with our shareholders who owned more than 5% of our outstanding ordinary shares before this offering, will, in the aggregate, beneficially own approximately % of our share capital. As a result, if these shareholders were to choose to act together, they would be able to control all matters submitted to our shareholders for approval, as well as our management and affairs. For example, these persons, if they choose to act together, would control the election of supervisory board directors and approval of any merger, consolidation or sale of all or substantially all of our assets.

***Provisions of our articles of association or Dutch corporate law might deter acquisition bids for us that might be considered favorable and prevent or frustrate any attempt to replace or remove the management board and supervisory board.***

Certain provisions of our articles of association may make it more difficult for a third party to acquire control of us or effect a change in our management board or supervisory board. These provisions include:

- the authorization of a class of preference shares that may be issued to a friendly party;
- staggered three-year terms of our supervisory directors;
- a provision that our managing directors and supervisory directors may only be removed at a general meeting of shareholders by a two-thirds majority of votes cast representing more than half of the issued share capital of the company (unless the removal was proposed by the supervisory board); and
- a requirement that certain matters, including an amendment of our articles of association, may only be brought to our shareholders for a vote upon a proposal by our management board that has been approved by our supervisory board.

***Our anti-takeover provision may prevent a beneficial change of control.***

We have adopted an anti-takeover measure pursuant to which our management board may, subject to supervisory board approval but without shareholder approval, issue (or grant the right to acquire) preference shares. We may issue an amount of preference shares up to 100% of our issued capital as per the moment immediately prior to the issuance of such preference shares.

In such event, the preference shares (or right to acquire preference shares) will be issued to a separate, newly established foundation. The preference shares will be issued to the foundation for their nominal value, of which only 25% will be due upon issuance. The voting rights of our shares are based on nominal value and, if our shares trade substantially in excess of nominal value, preference shares issued at nominal value can obtain significant voting power for a substantially reduced price and thus be used as a defensive measure. These preference shares will have both a liquidation and dividend preference over our ordinary shares and will accrue cash dividends at a fixed rate.



Our management board may issue these preference shares to protect us from influences that do not serve our best interests and threaten to undermine our continuity, independence and identity. These influences may include a third-party acquiring a significant percentage of our ordinary shares, the announcement of a public offer for our ordinary shares, other concentration of control over our ordinary shares or any other form of pressure on us to alter our strategic policies.

If the management board determines to issue the preference shares to such a foundation, the purpose clause in this foundation's articles of association will provide that it will act to serve the best interests of us, our associated business and all parties connected to us, by opposing any influences that conflict with these interests and threaten to undermine our continuity, independence and identity. This foundation will be structured to operate independently of us and we will not have direct control over or be able to influence the actions of the foundation.

***We do not expect to pay dividends in the foreseeable future.***

We have not paid any dividends since our incorporation. Even if future operations lead to significant levels of distributable profits, we currently intend that earnings, if any, will be reinvested in our business and that dividends will not be paid until we have an established revenue stream to support continuing dividends. Payment of future dividends to shareholders will, in addition, be at the discretion of the management board, subject to the approval of the supervisory board after taking into account various factors including our business prospects, cash requirements, financial performance, debt covenant limitations and new product development. Our current loan agreement with Hercules restricts our ability to pay dividends. In addition, payment of future dividends may be made only if our shareholders' equity exceeds the sum of our paid-in and called-up share capital plus the reserves required to be maintained by Dutch law or by our articles of association. Accordingly, investors cannot rely on dividend income from our ordinary shares and any returns on an investment in our ordinary shares will likely depend entirely upon any future appreciation in the price of our ordinary shares.

***We expect that only a relatively small percentage of our ordinary shares will be publicly traded following this offering, which may limit the liquidity of your investment and may have a material adverse effect on the price of our ordinary shares.***

After this offering, % of our ordinary shares will be held by parties other than our directors, senior management, existing shareholders holding 5% or more of our ordinary shares, and their respective affiliates. As a result, we expect that only a relatively small number of our ordinary shares will be actively traded in the public market following this offering. Reduced liquidity may have a material adverse effect on the price of our ordinary shares.

***You will not be able to trade our ordinary shares on any exchange outside the United States.***

Our ordinary shares will be listed only in the United States on the NASDAQ Global Market and we have no plans to list our ordinary shares in any other jurisdiction. As a result, a holder of our ordinary shares outside the United States may not be able to effect transactions in our ordinary shares as readily as the holder may if our securities were listed on an exchange in that holder's home jurisdiction.

***The sale of a substantial number of our ordinary shares following this offering may cause the market price of our ordinary shares to decline.***

Sales of a substantial number of shares in the public market may occur at any time after the expiration of the lock-up agreements described in the "Underwriting" section of this prospectus. Our sale or the resale by our shareholders of shares, or a market expectation of such sales, after this offering may cause the market price of our ordinary shares to decline. After this offering, we will have outstanding ordinary shares. Of these, the ordinary shares sold in this offering will be freely transferable without restriction. The remaining ordinary shares, or % of our outstanding shares after this offering, are currently

restricted as a result of securities laws or lock-up agreements but will be able to be sold, subject to any applicable volume limitations under U.S. federal securities laws with respect to affiliate sales, in the future as set forth in "Shares Eligible for Future Sale" and "Underwriting" below.

***We are an "emerging growth company," and the reduced disclosure requirements applicable to emerging growth companies may make our ordinary shares less attractive to investors.***

We are an "emerging growth company," as defined in the Jumpstart Our Business Startups Act of 2012, or the JOBS Act, and may remain an emerging growth company for up to five years. For so long as we remain an emerging growth company, we are permitted and intend to rely on exemptions from certain disclosure requirements that are applicable to other public companies that are not emerging growth companies. These exemptions include:

- being permitted to provide only two years of audited financial statements, in addition to any required unaudited interim financial statements, with correspondingly reduced "Management's Discussion and Analysis of Financial Condition and Results of Operations" disclosure;
- not being required to comply with the auditor attestation requirements in the assessment of our internal control over financial reporting; and
- not being required to comply with any requirement that may be adopted by the Public Company Accounting Oversight Board regarding mandatory audit firm rotation or a supplement to the auditor's report providing additional information about the audit and the financial statements;

We have taken advantage of reduced reporting burdens in this prospectus. In particular, in this prospectus, we have provided only two years of audited financial statements. We cannot predict whether investors will find our ordinary shares less attractive if we rely on these exemptions. If some investors find our ordinary shares less attractive as a result, there may be a less active trading market for our ordinary shares and our share price may be more volatile.

***We will be a foreign private issuer and, as a result, we will not be subject to U.S. proxy rules and will be subject to Exchange Act reporting obligations that, to some extent, are more lenient and less frequent than those of a U.S. domestic public company.***

Upon consummation of this offering, we will report under the Securities Exchange Act of 1934, as amended, or the Exchange Act, as a non-U.S. company with foreign private issuer status. Because we qualify as a foreign private issuer under the Exchange Act and although we are subject to Dutch laws and regulations with regard to such matters and intend to furnish quarterly financial information to the SEC, we are exempt from certain provisions of the Exchange Act that are applicable to U.S. domestic public companies, including:

- the sections of the Exchange Act regulating the solicitation of proxies, consents or authorizations in respect of a security registered under the Exchange Act;
- the sections of the Exchange Act requiring insiders to file public reports of their stock ownership and trading activities and liability for insiders who profit from trades made in a short period of time; and
- the rules under the Exchange Act requiring the filing with the Securities and Exchange Commission of quarterly reports on Form 10-Q containing unaudited financial and other specified information, or current reports on Form 8-K, upon the occurrence of specified significant events.

In addition, foreign private issuers are not be required to file their annual report on Form 20-F until 120 days after the end of each fiscal year, while U.S. domestic issuers that are accelerated filers are required to file their annual report on Form 10-K within 75 days after the end of each fiscal year. Foreign private issuers are also exempt from the Regulation Fair Disclosure, aimed at preventing issuers from making selective disclosures of material information. As a result of the above, you may not have the same protections afforded to shareholders of companies that are not foreign private issuers.

***We may lose our foreign private issuer status which would then require us to comply with the Exchange Act's domestic reporting regime and cause us to incur significant legal, accounting and other expenses.***

We are a foreign private issuer and therefore we are not required to comply with all of the periodic disclosure and current reporting requirements of the Exchange Act applicable to U.S. domestic issuers. In order to maintain our current status as a foreign private issuer, either:

- a majority of our ordinary shares must be either directly or indirectly owned of record by non-residents of the United States; or
- a majority of our executive officers or directors may not be United States citizens or residents, more than 50 percent of our assets cannot be located in the United States and our business must be administered principally outside the United States.

If we lost this status, we would be required to comply with the Exchange Act reporting and other requirements applicable to U.S. domestic issuers, which are more detailed and extensive than the requirements for foreign private issuers.

We may also be required to make changes in our corporate governance practices in accordance with various SEC and NASDAQ rules. The regulatory and compliance costs to us under U.S. securities laws if we are required to comply with the reporting requirements applicable to a U.S. domestic issuer may be significantly higher than the cost we would incur as a foreign private issuer. As a result, we expect that a loss of foreign private issuer status would increase our legal and financial compliance costs and would make some activities highly time consuming and costly. We also expect that if we were required to comply with the rules and regulations applicable to U.S. domestic issuers, it would make it more difficult and expensive for us to obtain director and officer liability insurance, and we may be required to accept reduced coverage or incur substantially higher costs to obtain coverage. These rules and regulations could also make it more difficult for us to attract and retain qualified members of our supervisory board.

***We have broad discretion in the use of the net proceeds from this offering and may not use them effectively.***

Our management will have broad discretion in the application of the net proceeds from this offering and could spend the proceeds in ways that do not improve our results of operations or enhance the value of our ordinary shares. The failure by our management to apply these funds effectively could result in financial losses that could have a material adverse effect on our business, cause the price of our ordinary shares to decline and delay the development of our product candidates. Pending their use, we may invest the net proceeds from this offering in a manner that does not produce income or that loses value.

***We will incur increased costs as a result of operating as a public company, and our management will be required to devote substantial time to new compliance initiatives and corporate governance practices.***

As a public company, and particularly after we are no longer an emerging growth company, we will incur significant legal, accounting and other expenses that we did not incur as a private company. We currently estimate that we will incur incremental annual costs of approximately \$1.2 million associated with operating as a public company, although it is possible that our actual incremental annual costs will be higher than we currently estimate. The Sarbanes-Oxley Act of 2002, the Dodd-Frank Wall Street Reform and Consumer Protection Act, the listing requirements of The NASDAQ Global Market and other applicable securities rules and regulations impose various requirements on public companies, including establishment and maintenance of effective disclosure and financial controls and corporate governance practices. Our management and other personnel will need to devote a substantial amount of time to these compliance initiatives. Moreover, these rules and regulations will increase our legal and financial compliance costs and will make some activities more time-consuming and costly. For example, we expect that these rules and regulations may make it more difficult and more expensive for us to obtain director and officer liability

insurance, which in turn could make it more difficult for us to attract and retain qualified members of our supervisory board.

These rules and regulations are often subject to varying interpretations, in many cases due to their lack of specificity, and, as a result, their application in practice may evolve over time as new guidance is provided by regulatory and governing bodies. This could result in continuing uncertainty regarding compliance matters and higher costs necessitated by ongoing revisions to disclosure and governance practices and control environment process improvements.

***If we fail to implement and maintain an effective system of internal controls, we may be unable to accurately report our results of operations or prevent fraud or fail to meet our reporting obligations, and investor confidence and the market price of our ordinary shares may be materially and adversely affected.***

Prior to this offering, we were a private company with limited accounting personnel and other resources with which to address our internal controls and procedures. Our independent registered public accounting firm has not conducted an audit of our internal control over financial reporting. However, in connection with the audit of our consolidated financial statements as of and for year ended December 31, 2012 and the review of our consolidated financial statements as of and for the nine months ended September 30, 2013, we and our independent registered public accounting firm identified three material weaknesses in our internal control over financial reporting. A significant deficiency is a control deficiency, or a combination of control deficiencies, that adversely affects our ability to initiate, authorize, record, process, or report external financial data reliably in accordance with IFRS such that there is more than a remote likelihood that a misstatement of our annual or interim financial statements that is more than inconsequential will not be prevented or detected by our employees. A material weakness is a significant deficiency, or combination of significant deficiencies, that results in more than a remote likelihood that a material misstatement of our annual or interim financial statement will not be presented or detected by our employees. In response, we have begun the process of evaluating our internal control over financial reporting, although we may not complete our review until after this offering is completed. We have also taken several remedial actions to address these material weaknesses. For details, see "Management's Discussion and Analysis of Financial Condition and Results of Operations—Internal Control Over Financial Reporting."

Furthermore, it is possible that, had our independent registered public accounting firm conducted an audit of our internal control over financial reporting, such firm might have identified additional material weaknesses and deficiencies. Upon the completion of this offering, we will become a public company in the United States subject to the Sarbanes-Oxley Act of 2002. Section 404 of the Sarbanes-Oxley Act of 2002, or Section 404, will require that we include a report of management on our internal control over financial reporting in our annual report on Form 20-F beginning with our annual report for the fiscal year ending December 31, 2015. In addition, once we cease to be an "emerging growth company" as such term is defined in the JOBS Act, our independent registered public accounting firm must attest to and report on the effectiveness of our internal control over financial reporting. Our management may conclude that our internal control over financial reporting is not effective. Moreover, even if our management concludes that our internal control over financial reporting is effective, our independent registered public accounting firm, after conducting its own independent testing, may issue a report that is qualified if it is not satisfied with our internal controls or the level at which our controls are documented, designed, operated or reviewed, or if it interprets the relevant requirements differently from us. In addition, after we become a public company, our reporting obligations may place a significant strain on our management, operational and financial resources and systems for the foreseeable future. We may be unable to timely complete our evaluation testing and any required remediation.

During the course of documenting and testing our internal control procedures, in order to satisfy the requirements of Section 404, we may identify other weaknesses and deficiencies in our internal control over financial reporting. In addition, if we fail to maintain the adequacy of our internal control over financial reporting, as these standards are modified, supplemented or amended from time to time, we may not be

able to conclude on an ongoing basis that we have effective internal control over financial reporting in accordance with Section 404. If we fail to achieve and maintain an effective internal control environment, we could suffer material misstatements in our financial statements and fail to meet our reporting obligations, which would likely cause investors to lose confidence in our reported financial information. This could in turn limit our access to capital markets, harm our results of operations, and lead to a decline in the trading price of our common shares. Additionally, ineffective internal control over financial reporting could expose us to increased risk of fraud or misuse of corporate assets and subject us to potential delisting from the stock exchange on which we list, regulatory investigations and civil or criminal sanctions. We may also be required to restate our financial statements for prior periods.

***You will experience immediate and substantial dilution in the net tangible book value of ordinary shares purchased.***

The initial public offering price per ordinary share will be substantially higher than the net tangible book value per ordinary share prior to the offering. Consequently, when you purchase ordinary shares in the offering at the assumed initial public offering price, you will incur immediate dilution of \$ \_\_\_\_\_ per ordinary share. See "Dilution."

***We intend to rely on NASDAQ Stock Market rules that permit us to comply with applicable Dutch corporate governance practices, rather than the corresponding domestic U.S. corporate governance practices, and therefore your rights as a shareholder will differ from the rights you would have as a shareholder of a domestic U.S. issuer.***

As a foreign private issuer whose ordinary shares are listed on the NASDAQ Global Market, we are permitted in certain cases to follow Dutch corporate governance practices instead of the corresponding requirements of the NASDAQ Marketplace Rules. A foreign private issuer that elects to follow a home country practice instead of NASDAQ requirements must submit to NASDAQ in advance a written statement from an independent counsel in such issuer's home country certifying that the issuer's practices are not prohibited by the home country's laws. In addition, a foreign private issuer must disclose in its annual reports filed with the Securities and Exchange Commission each such requirement that it does not follow and describe the home country practice followed instead of any such requirement. We intend to follow Dutch corporate governance practices with regard to the quorum requirements applicable to meetings of shareholders and the provision of proxy statements for general meetings of shareholders, rather than the corresponding domestic U.S. corporate governance practices. In accordance with Dutch law and generally accepted business practices, our articles of association do not provide quorum requirements generally applicable to general meetings of shareholders. Although we do intend to provide shareholders with an agenda and other relevant documents for the general meeting of shareholders, Dutch law does not have a regulatory regime for the solicitation of proxies and the solicitation of proxies is not a generally accepted business practice in the Netherlands. Accordingly, our shareholders may not be afforded the same protection as provided under NASDAQ's corporate governance rules.

***We do not comply with all the provisions of the Dutch Corporate Governance Code. This may affect your rights as a shareholder.***

As a Dutch company we are subject to the Dutch Corporate Governance Code, or DCGC. The DCGC contains both principles and best practice provisions for management boards, supervisory boards, shareholders and general meetings of shareholders, financial reporting, auditors, disclosure, compliance and enforcement standards. The DCGC applies to all Dutch companies listed on a government-recognized stock exchange, whether in the Netherlands or elsewhere, including the NASDAQ Global Market. The principles and best practice provisions apply to our management board and supervisory board, in relation to their role and composition, conflicts of interest and independence requirements, board committees and remuneration, shareholders and the general meeting of shareholders, for example, regarding anti-takeover protection and obligations of the company to provide information to its shareholders; and financial reporting, including

external auditor and internal audit requirements. We do not comply with all the provisions of the DCGC. This may affect your rights as a shareholder and you may not have the same level of protection as a shareholder in a Dutch company that fully complies with the DCGC.

## Risks for U.S. Holders

***We cannot assure you that we will not be classified as a passive foreign investment company for any taxable year, which may result in adverse U.S. federal income tax consequence to U.S. holders.***

Based on our estimated gross income and average value of our gross assets, taking into account the assumed initial public offering price of our shares in this offering and the expected price of our shares following the offering, and the nature of our business, we do not expect to be considered a "passive foreign investment company," or PFIC, for U.S. federal income tax for the 2013 tax year or in the foreseeable future. A corporation organized outside the United States generally will be classified as a PFIC for U.S. federal income tax purposes in any taxable year in which at least 75% of its gross income is passive income or on average at least 50% of the gross value of its assets is attributable to assets that produce passive income or are held for the production of passive income. Passive income for this purpose generally includes dividends, interest, royalties, rents and gains from commodities and securities transactions. Our status in any taxable year will depend on our assets and activities in each year, and because this is a factual determination made annually after the end of each taxable year, there can be no assurance that we will not be considered a PFIC for the current taxable year or any future taxable year. The market value of our assets may be determined in large part by reference to the market price of our ordinary shares, which is likely to fluctuate after the offering, and may fluctuate considerably given that market prices of technology companies have been especially volatile. If we were to be treated as a PFIC for any taxable year during which a U.S. holder held our ordinary shares, however, certain adverse U.S. federal income tax consequences could apply to the U.S. holder. See "Taxation—Taxation in the United States—U.S. federal income tax consequences to U.S. holders—Passive foreign investment company considerations."

***Any U.S. or other foreign judgments you may obtain against us may be difficult to enforce against us in the Netherlands.***

We are incorporated under the laws of the Netherlands. We currently have only limited operations in the United States. Most of our assets are currently located in the Netherlands.

The majority of our managing directors, supervisory directors and senior management reside outside the United States. As a result, it may not be possible for investors to effect service of process within the United States upon such persons or to enforce against them or us in U.S. courts, including judgments predicated upon the civil liability provisions of the federal securities laws of the United States. In addition, it is not clear whether a Dutch court would impose civil liability on us or any of our managing directors or supervisory directors in an original action based solely upon the federal securities laws of the United States brought in a court of competent jurisdiction in the Netherlands.

The United States and the Netherlands currently do not have a treaty providing for the reciprocal recognition and enforcement of judgments, other than arbitration awards, in civil and commercial matters. Consequently, a final judgment for payment given by a court in the United States, whether or not predicated solely upon U.S. securities laws, would not automatically be recognized or enforceable in the Netherlands. In order to obtain a judgment which is enforceable in the Netherlands, the party in whose favor a final and conclusive judgment of the U.S. court has been rendered will be required to file its claim with a court of competent jurisdiction in the Netherlands. Such party may submit to the Dutch court the final judgment rendered by the U.S. court. If and to the extent that the Dutch court finds that the jurisdiction of the U.S. court has been based on grounds which are internationally acceptable and that proper legal procedures have been observed, the Dutch court will, in principle, give binding effect to the judgment of the U.S. court, unless such judgment contravenes principles of public policy of the Netherlands. Dutch courts may deny

the recognition and enforcement of punitive damages or other awards. Moreover, a Dutch court may reduce the amount of damages granted by a U.S. court and recognize damages only to the extent that they are necessary to compensate actual losses or damages. Enforcement and recognition of judgments of U.S. courts in the Netherlands are solely governed by the provisions of the Dutch Civil Procedure Code.

Based on the lack of a treaty as described above, U.S. investors may not be able to enforce against us or our management board or supervisory board members, representatives or certain experts named herein who are residents of the Netherlands or countries other than the United States any judgments obtained in U.S. courts in civil and commercial matters, including judgments under the U.S. federal securities laws.

***The rights and responsibilities of our shareholders are governed by Dutch law and differ in some important respects from the rights and responsibilities of shareholders under U.S. law.***

Our corporate affairs are governed by our articles of association and by the laws governing companies incorporated in the Netherlands. The rights of our shareholders and the responsibilities of members of our supervisory board under Dutch law are different than under the laws of some U.S. jurisdictions. In the performance of its duties, our supervisory board is required by Dutch law to consider the interests of uniQure, its shareholders, its employees and other stakeholders and not only those of our shareholders. Also, as a Dutch company, we are not required to solicit proxies or prepare proxy statements for general meetings of shareholders. Dutch law does not have a regulatory regime for U.S.- style proxy solicitations and, even though Dutch law accommodates voting by proxy, the solicitation of proxies is not a widely used business practice in the Netherlands.

In addition, the rights of holders of shares and many of the rights of shareholders as they relate to, for example, the exercise of shareholder rights, are governed by Dutch law and our articles of association and differ from the rights of shareholders under U.S. law. For example, Dutch law does not grant appraisal rights to a company's shareholders who wish to challenge the consideration to be paid upon a merger or consolidation of the company. See "Description of Share Capital—Differences in Corporate Law."

## SPECIAL NOTE REGARDING FORWARD-LOOKING STATEMENTS AND INDUSTRY DATA

This prospectus contains forward-looking statements that involve substantial risks and uncertainties. All statements contained in this prospectus, other than statements of historical fact, including statements regarding our strategy, future operations, future financial position, future revenues, projected costs, prospects, plans and objectives of management, are forward-looking statements. The words "anticipate," "believe," "estimate," "expect," "intend," "may," "plan," "predict," "project," "target," "potential," "will," "would," "could," "should," "continue," and similar expressions are intended to identify forward-looking statements, although not all forward-looking statements contain these identifying words. The forward-looking statements in this prospectus include, among other things, statements about:

- the timing of commencement and receipt of data in our planned clinical trials;
- the timing of the ongoing and planned clinical trials conducted by our collaborators and other third parties;
- our ongoing and planned discovery and development of product candidates;
- our expectations regarding the timing or likelihood of regulatory filings and approvals for our product candidates;
- our ability to expand our sales, marketing and medical affairs infrastructure;
- our ability to successfully commercialize Glybera and our product candidates;
- the potential advantages of Glybera and our product candidates;
- our estimates regarding the market opportunities for our product candidates;
- the rate and degree of market acceptance and clinical benefit of Glybera and our product candidates;
- our expectations regarding milestone, royalty and expense reimbursement payments under our licensing arrangements;
- our estimates of the net amount we will retain from sales of Glybera;
- the timing and cost of the build-out of our manufacturing facility in Lexington, Massachusetts;
- our ability to establish and maintain collaborations;
- our ability to develop, acquire or in-license additional product candidates and other key intellectual property;
- our future intellectual property position; and
- our estimates regarding expenses, future revenues, capital requirements and needs for additional financing.

We may not actually achieve the plans, intentions or expectations disclosed in our forward-looking statements, and you should not place undue reliance on our forward-looking statements. Actual results or events could differ materially from the plans, intentions and expectations disclosed in the forward-looking statements we make. We have included important factors in the cautionary statements included in this prospectus, particularly in the "Risk Factors" section, that we believe could cause actual results or events to differ materially from the forward-looking statements that we make. Our forward-looking statements do not reflect the potential impact of any future acquisitions, mergers, dispositions, joint ventures or investments we may make.

You should read this prospectus and the documents that we have filed as exhibits to the registration statement of which this prospectus is a part completely and with the understanding that our actual future results may be materially different from what we expect. We do not assume any obligation to update any forward-looking statements.

This prospectus includes statistical and other industry and market data that we obtained from industry publications and research, surveys and studies conducted by third parties. Industry publications and third party research, surveys and studies generally indicate that their information has been obtained from sources believed to be reliable, although they do not guarantee the accuracy or completeness of such information. Although we believe these industry publications and third party research, surveys and studies are reliable, we have not independently verified such data.



## USE OF PROCEEDS

We estimate that we will receive net proceeds from this offering of \$                      million (€                      million), based upon an assumed initial public offering price of \$                      per ordinary share (the midpoint of the estimated price range set forth on the cover page of this prospectus), after deducting underwriting discounts and any offering expenses payable by us. If the underwriters exercise their options to purchase additional shares, we estimate that the net proceeds of the offering will be \$                      million (€                      million).

A \$1.00 increase (decrease) in the assumed initial public offering price of \$                      per ordinary share would increase (decrease) the net proceeds to us from this offering by approximately \$                      million, assuming the number of ordinary shares offered by us, as set forth on the cover page of this prospectus, remains the same and after deducting underwriting discounts and commissions and estimated offering expenses payable by us. Similarly, each increase (decrease) of one million ordinary shares in the number of ordinary shares offered by us would increase (decrease) the net proceeds to us from this offering by approximately \$                      million, assuming the assumed initial public offering price remains the same and after deducting underwriting discounts and commissions and estimated offering expenses payable by us.

As of September 30, 2013, we had cash and cash equivalents of \$31.4 million. We currently estimate that we will use the net proceeds from this offering, together with our cash on hand, as follows:

- approximately \$15.0 million to complete the building out and equipping of our manufacturing facility in Lexington, Massachusetts;
- approximately \$                      million to support our further clinical development of Glybera, and our application for marketing approval of Glybera and preparation for potential commercial launch in the United States;
- approximately \$                      million to fund our planned Phase I/II clinical trial of AMT-060 in hemophilia B;
- approximately \$                      million to advance the development of our other product candidates; and
- the remainder for working capital and for general corporate purposes, including service on our indebtedness and potentially for acquisitions or investments in other businesses, technologies or product candidates.

This expected use of net proceeds from this offering represents our intentions based upon our current plans and business conditions, which could change in the future as our plans and business conditions evolve. The amounts and timing of our actual expenditures may vary significantly depending on numerous factors, including the progress of our development, the status of and results from clinical trials, as well as any collaborations that we may enter into with third parties for our product candidates, and any unforeseen cash needs. As a result, our management retains broad discretion over the allocation of the net proceeds from this offering. We have no current agreements, commitments or understandings for any material acquisitions or licenses of any products, businesses or technologies.

Pending our use of the net proceeds from this offering, we intend to invest the net proceeds in a variety of capital preservation investments, including term deposits, short-term, investment-grade, interest-bearing instruments and U.S. government securities.

## **DIVIDEND POLICY**

We have never declared or paid any dividends on our ordinary shares, and we currently do not plan to declare dividends on our ordinary shares in the foreseeable future. Under Dutch law, we may only pay dividends if our shareholders' equity exceeds the sum of the paid-up and called-up share capital plus the reserves required to be maintained by Dutch law or by our articles of association. In addition, our loan agreement with Hercules contains, and any other loan facilities that we may enter into may contain, restrictions on our ability, or that of our subsidiaries, to pay dividends. Subject to such restrictions, a proposal for the payment of cash dividends in the future, if any, will be at the discretion of our management board, subject to the approval of our supervisory board, and will depend upon such factors as earnings levels, capital requirements, contractual restrictions, our overall financial condition and any other factors deemed relevant by our management board.

## CAPITALIZATION

The following table sets forth our cash and cash equivalents and capitalization as of September 30, 2013:

- on an actual basis; and
- on an as adjusted basis to give effect to the sale of \_\_\_\_\_ ordinary shares by us in this offering, assuming an initial public offering price of \$ \_\_\_\_\_ per ordinary share, the midpoint of the price range set forth on the cover page of this prospectus, and after deducting the estimated underwriting discounts and commissions and estimated offering expenses payable by us and the application of such net proceeds as described under "Use of Proceeds."

This table should be read with our consolidated financial statements and the related notes and "Management's Discussion and Analysis of Financial Condition and Results of Operations."

(in thousands, except share and per share data)	AS OF SEPTEMBER 30, 2013	
	ACTUAL	AS ADJUSTED <sup>(1)</sup>
Cash and cash equivalents	€ 31,427	€ _____
Total debt	8,456	_____
Shareholders' equity:		
Share capital		
Ordinary shares	609	_____
Share premium	142,444	_____
Other reserves	5,924	_____
Accumulated deficit	(137,656)	_____
Total shareholders' equity	11,321	_____
Total capitalization	€ 19,777	€ _____

- <sup>(1)</sup> Each \$1.00 increase or decrease in the assumed initial public offering price of \$ \_\_\_\_\_ per ordinary share would increase or decrease, respectively, the amount of cash and cash equivalents, working capital, total assets and total shareholders' equity by \$ \_\_\_\_\_ million, assuming the number of ordinary shares offered by us, as set forth on the cover page of this prospectus, remains the same and after deducting the underwriting discounts and commissions and estimated offering expenses payable by us. We may also increase or decrease the number of ordinary shares we are offering. An increase or decrease of 1,000,000 in the number of ordinary shares we are offering would increase or decrease, respectively, the amount of cash, cash equivalents and short-term investments, working capital, total assets and stockholders' equity by approximately \$ \_\_\_\_\_ million, assuming the assumed initial public offering price per ordinary share, as set forth on the cover page of this prospectus, remains the same. The as adjusted information is illustrative only, and we will adjust this information based on the actual initial public offering price and other terms of this offering determined at pricing.

The table above excludes:

- 8,451,110 ordinary shares issuable upon the exercise of options outstanding as of September 30, 2013 at a weighted average exercise price of €0.78 per share. See "Management—Compensation—Share Options";
- up to \_\_\_\_\_ ordinary shares reserved for future issuance under our equity incentive plans following this offering; and
- 854,036 ordinary shares issuable upon the exercise of warrants outstanding as of September 30, 2013 at an exercise price of €2.02 per ordinary share.

## DILUTION

If you invest in our ordinary shares, your interest will be diluted to the extent of the difference between the initial public offering price per share and the net tangible book value per share after this offering. Our net tangible book value as of September 30, 2013, was €                      million (\$                      million), or €                      (\$                      ) per share. Net tangible book value per share represents the amount of our total tangible assets less our total liabilities, divided by the number of shares outstanding as of September 30, 2013.

After giving effect to the sale by us of                      ordinary shares in this offering at an assumed initial public offering price of \$                      per ordinary share (€                      per share) (the midpoint of the estimated price range on the cover of this prospectus), and after deducting underwriting discounts and commissions and estimated offering expenses payable by us, our as adjusted net tangible book value as of September 30, 2013, would have been €                      million (\$                      million), or €                      per share (\$                      per share). This amount represents an immediate increase in net tangible book value of €                      per share (\$                      per share) to our existing shareholders and an immediate dilution in net tangible book value of €                      per share (\$                      per share), or                      % per share, to new investors purchasing ordinary shares in this offering at the assumed initial public offering price. We determine dilution by subtracting the pro forma net tangible book value per share after this offering from the amount of cash that a new investor paid for an ordinary share. The following table illustrates this dilution on a per share basis:

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Assumed initial public offering price per share	\$	€
Net tangible book value per share as of September 30, 2013		
Increase per share attributable to new investors in this offering		
As adjusted net tangible book value per share as of September 30, 2013 after giving effect to this offering		
Dilution per share to new investors		

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A \$1.00 increase (decrease) in the assumed initial public offering price of \$                      per ordinary share would increase (decrease) the pro forma net tangible book value, as adjusted to give effect to this offering, by \$                      per ordinary share and increase (decrease) the dilution to new investors by \$                      per ordinary share, assuming the number of shares offered by us, as set forth on the cover page of this prospectus, remains the same, and after deducting underwriting discounts and commissions and estimated expenses payable by us. If the underwriters exercise their option to purchase additional ordinary shares from us in full, the pro forma net tangible book value per share of our shares, as adjusted to give effect to this offering, would be \$                      per share, and the dilution in pro forma net tangible book value per share to investors in this offering would be \$                      per ordinary share.

The table below summarizes as of September 30, 2013, the number of our ordinary shares, the total consideration and the average price per share (a) paid to us by existing shareholders and (b) to be paid by new investors purchasing our ordinary shares in this offering at an assumed initial public offering price of

\$ \_\_\_\_\_ per share, the midpoint of the estimated price range set forth on the cover page of this prospectus, before deducting underwriting discounts and commissions and estimated offering expenses.

	SHARES PURCHASED		TOTAL CONSIDERATION		AVERAGE PRICE PER SHARE	
	NUMBER	PERCENT	AMOUNT	PERCENT		
Existing shareholders		%	\$	€	%	\$
New investors						€
Total		100.0%			100.0%	

The total number of shares reflected in the discussion and tables above is based on 60,948,978 ordinary shares outstanding as of September 30, 2013.

The table above excludes:

- 8,451,110 shares issuable upon the exercise of options outstanding as of as of September 30, 2013 at a weighted average exercise price of €0.78 per share. See "Management—Compensation—Share Options";
- up to \_\_\_\_\_ shares reserved for future issuance under our equity incentive plans following this offering; and
- 854,036 ordinary shares issuable upon the exercise of warrants outstanding as of September 30, 2013 at an exercise price of €2.02 per share.

If the underwriters exercise their option to purchase additional shares in full, the number of ordinary shares beneficially owned by existing shareholders would decrease to approximately \_\_\_\_\_, or approximately \_\_\_\_\_ % of the total number of ordinary shares outstanding after this offering, and the number of shares held by new investors will be increased to \_\_\_\_\_ shares, or approximately \_\_\_\_\_ % of the total number of ordinary shares outstanding after this offering.

To the extent options are exercised and awards are granted under these plans, there may be dilution to our shareholders. We may also choose to raise additional capital due to market conditions or strategic considerations even if we believe we have sufficient funds for our current or future operating plans. To the extent that we raise additional capital through the sale of equity or convertible debt securities, the issuance of these securities could result in further dilution to our shareholders.

## SELECTED CONSOLIDATED FINANCIAL DATA

The following selected consolidated financial data as of and for the years ended December 31, 2011 and 2012 have been derived from our consolidated financial statements included elsewhere in this prospectus.

The following selected consolidated financial data as of and for the nine months ended September 30, 2012 and 2013 have been derived from our unaudited consolidated financial statements included elsewhere in this prospectus. The unaudited financial statements have been prepared on the same basis as the audited financial statements and, in the opinion of management, reflect all adjustments, which include only normal recurring adjustments, necessary to state fairly our financial position as of September 30, 2013 and the results of operations for the nine months ended September 30, 2012 and 2013.

Our historical results for any prior period are not necessarily indicative of results to be expected in any future period, and our interim period results are not necessarily indicative of results to be expected for a full year or any other interim period. The information set forth below should be read in conjunction with the "Management's Discussion and Analysis of Financial Condition and Results of Operations" section of this prospectus and with our consolidated financial statements and notes thereto included elsewhere in this prospectus.

### Consolidated Statements of Comprehensive Income Data:

€ in thousands (except share and per share data)	YEAR ENDED DECEMBER 31,		NINE MONTHS ENDED SEPTEMBER 30,	
	2011	2012	2012	2013
<b>Revenues:</b>				
License revenues	—	—	—	€ 220
Collaboration revenues	—	—	—	1,831
<b>Total revenues</b>	—	—	—	2,051
Cost of goods sold	—	—	—	(800)
<b>Gross profit</b>	—	—	—	1,251
Other income	€ 2,192	€ 649	€ 315	686
Research and development expenses	(15,500)	(10,231)	(5,690)	(9,856)
Selling, general and administrative expenses	(3,807)	(4,564)	(4,438)	(7,612)
Other losses, net	(26)	(45)	(82)	(269)
<b>Operating result</b>	<b>(17,141)</b>	<b>(14,191)</b>	<b>(9,985)</b>	<b>(15,800)</b>
Finance income	277	22	16	48
Finance expense	(436)	(547)	(545)	(4,676)
<b>Net loss</b>	<b>(17,300)</b>	<b>(14,716)</b>	<b>(10,424)</b>	<b>(20,428)</b>
<b>Basic and diluted loss per share</b>	<b>(0.73)</b>	<b>(0.34)</b>	<b>(0.25)</b>	<b>(0.39)</b>
Weighted average shares outstanding used in computing per share amounts:				
Basic and diluted	23,549	43,187	42,156	52,972

The following table sets forth selected balance sheet data as of the dates indicated:

**Consolidated Balance Sheet Data:**

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(€ in thousands)	AS OF DECEMBER 31,			AS OF
	2010	2011	2012	SEPTEMBER 30, 2013
Cash and cash equivalents	€ 17,859	€ 1,100	€ 263	€ 31,427
Total assets	22,703	5,804	5,567	43,671
Total debt	4,621	4,544	1,498	8,456
Accumulated deficit	(88,205)	(105,505)	(117,234)	(137,656)
Total shareholders' equity (deficit)	13,659	(2,593)	(448)	11,321

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## EXCHANGE RATE INFORMATION

Our business to date has been conducted primarily in the European Union, and we prepare our consolidated financial statements in euros. In this prospectus, translations from euros to U.S. dollars were made at the rate of €0.741 to \$1.00, the official exchange rate quoted as of September 30, 2013 by the European Central Bank. Such U.S. dollar amounts are not necessarily indicative of the amounts of U.S. dollars that could actually have been purchased upon exchange of euros at the dates indicated. On December 18, 2013, the exchange rate was €0.727 to \$1.00. The following table presents information on the exchange rates between the euro and the U.S. dollar for the periods indicated:

(€ per U.S. dollar)	PERIOD END	AVERAGE	LOW	HIGH
<b>Year Ended December 31:</b>				
2008	0.719	0.680	0.625	0.803
2009	0.694	0.717	0.661	0.796
2010	0.748	0.754	0.687	0.837
2011	0.773	0.718	0.672	0.776
2012	0.758	0.778	0.743	0.827
2013 (through December 18)	0.727	0.754	0.724	0.783
<b>Month Ended:</b>				
June 2013	0.764	0.758	0.745	0.767
July 2013	0.753	0.765	0.752	0.780
August 2013	0.755	0.751	0.746	0.757
September 2013	0.740	0.749	0.738	0.762
October 2013	0.733	0.733	0.724	0.741
November 2013	0.735	0.741	0.735	0.748
December 2013 (through December 18)	0.727	0.730	0.726	0.739

This prospectus also contains amounts that we have paid or may be required to pay in Canadian dollars. On December 13, 2013, the exchange rate quoted by the Federal Reserve Bank of New York was C\$1.059 to \$1.00.



## MANAGEMENT'S DISCUSSION AND ANALYSIS OF FINANCIAL CONDITION AND RESULTS OF OPERATIONS

*You should read the following discussion and analysis of our financial condition and results of operations together with the information under "Selected Financial Information" and our consolidated audited and unaudited interim financial statements, including the notes thereto, included elsewhere in this prospectus. The following discussion is based on our financial statements prepared in accordance with International Financial Reporting Standards, or IFRS, as issued by the International Accounting Standards Board, or IASB, which might differ in material respects from generally accepted accounting principles in other jurisdictions. The following discussion includes forward-looking statements that involve risks, uncertainties and assumptions. Our actual results may differ materially from those anticipated in these forward-looking statements as a result of many factors, including but not limited to those described under "Risk Factors".*

### Overview

We are a leader in the field of gene therapy and have developed the first and currently the only gene therapy product to receive regulatory approval in the European Union. Our first product, Glybera, was approved by the European Commission in October 2012 under exceptional circumstances for the treatment of a subset of patients with lipoprotein lipase deficiency, or LPLD, a potentially life-threatening, orphan metabolic disease. We expect to launch Glybera commercially in selected European countries in the first half of 2014 through our collaboration with Chiesi, which we entered into in April 2013. We retain full commercial rights to Glybera in the United States. In August and December 2013, we met with the FDA to discuss the regulatory pathway for Glybera in the United States and we plan to file an IND with the FDA for Glybera in the first half of 2014. We are developing a pipeline of additional AAV-based gene therapies through multiple collaborations designed to accelerate the development and commercialization of these programs. We develop our gene therapies using our innovative, modular technology platform, including our proprietary, cost-effective manufacturing process.

Our business was founded in 1998 by scientists who were investigating LPLD at the Academic Medical Center of the University of Amsterdam, or the AMC. In our early years we received funding and subsidized rent from the AMC, government grants, income for cGMP contract manufacturing of biologics for third parties, and small amounts of equity financing. Since our first institutional venture capital financing in 2006, we have funded our operations primarily through private and public placements of equity securities, and other convertible debt securities, in the aggregate amount of €137.5 million (\$179.9 million). During this period, we have also received total other income, consisting principally of government grants and subsidies, of €5.9 million, and total nonrefundable collaboration funding of €17.0 million. Our predecessor entity, Amsterdam Molecular Therapeutics (AMT) N.V., or AMT, completed an initial public offering of its ordinary shares on Euronext Amsterdam in 2007. We acquired the business of AMT in the first half of 2012, as described below.

The total amounts described above include the following funds received in 2013:

- €12.0 million in convertible loan financing, which we received in the first quarter of 2013, and which was converted into equity in July 2013;
- \$10.0 million (€7.5 million) in venture debt financing, which we received in the second quarter of 2013;
- €17.0 million in upfront payments from Chiesi under our collaboration agreements for Glybera and hemophilia B, which we received in July 2013; and
- €14.0 million in equity funding from Chiesi, which we received in July 2013.

As of September 30, 2013, we had cash and cash equivalents of €31.4 million. To date, we have not generated any revenues from royalties or product sales. We do not expect to generate royalty or revenues from product sales prior to the commercial launch of Glybera by Chiesi.

We had a net loss of €20.4 million in the first nine months of 2013, €14.7 million in 2012 and €17.3 million in 2011. As of September 30, 2013, we had an accumulated deficit of €138 million. We anticipate that our expenses will increase substantially in the future as we:

- complete our EMA-mandated post-approval clinical trial of Glybera and implement an LPLD patient registry;
- conduct a confirmatory clinical trial of Glybera, either as part of the EMA-mandated post-approval clinical trial or separately, to obtain data needed to file a BLA for Glybera with the FDA;
- seek marketing approval for Glybera in the United States and other countries;
- initiate a Phase I/II clinical trial of AMT-060 for hemophilia B in collaboration with Chiesi;
- advance the preclinical and clinical development of our other product candidates, most of which are at relatively early stages of development, and seek to discover and develop additional product candidates;
- seek marketing approval for any product candidates that successfully complete clinical trials;
- establish a sales, marketing and medical affairs infrastructure in the United States;
- complete the building out and equipping of our manufacturing facility in Lexington, Massachusetts to expand our manufacturing capabilities for Glybera and our pipeline of product candidates;
- fund the ongoing operations of our Lexington facility;
- maintain, expand and protect our intellectual property portfolio, including in-licensing additional intellectual property rights from third parties;
- hire additional personnel, particularly in our manufacturing, research, clinical development, medical affairs, commercial and quality control groups;
- add operational, financial and management information systems and related finance and compliance personnel; and
- operate as a public company.

#### **Accounting for our Corporate Reorganization and Strategic Restructuring**

At the end of 2011, following the initial rejection of the application for marketing approval for Glybera in the European Union, AMT initiated a strategic restructuring in order to reduce its cost base, conserve resources and improve its financial position. As part of this effort, AMT significantly reduced personnel, programs and expenditures. As a result, we lost a number of employees, including employees with an extensive understanding of our clinical programs as well as our regulatory and financial affairs. AMT implemented a strategic restructuring in the fourth quarter of 2011, as a result of which total staff was reduced from 92 to 49. AMT incurred significant restructuring expenses in connection with this reduction in staff, which were recorded in full during the fourth quarter of 2011. Since that time, we have hired a number of new staff. As of September 30, 2013, we had a total of 79 employees and engaged 33 consultants and contract workers.

In the first half of 2012, we completed a corporate reorganization pursuant to which uniQure acquired the entire business of the AMT group. Pursuant to IFRS, this reorganization was treated as a reverse acquisition of AMT and accordingly, for accounting purposes, AMT was treated as the acquirer. As a result, the historical financial statements of AMT are treated as the financial statements of uniQure. See Note 1 to the audited consolidated financial statements included elsewhere in this prospectus for further details.

At the time AMT originally prepared its audited financial statements for 2011, the business of AMT was in liquidation and therefore the related financial statements were prepared on a liquidation basis rather than a going concern basis. As of December 31, 2011, it was regarded as probable that the business and assets of AMT would be disposed of, and therefore AMT's assets and liabilities were recorded as assets and liabilities held for sale and its operating results were recorded as discontinued operations. Following the corporate reorganization described above, we restated the financial information of AMT as of and for the year ended

December 31, 2011 on a going concern basis. See Note 5 to the 2012 audited consolidated financial statements included elsewhere in this prospectus for further details.

## **Collaboration and License Agreements**

### ***Chiesi Agreements***

In April 2013 we entered into two collaboration agreements with Chiesi. In July 2013, we received an aggregate of €17.0 million in upfront payments from Chiesi under these agreements, as well as a €14.0 million investment in our ordinary shares.

#### ***Glybera agreement***

Under the Glybera agreement, we granted Chiesi the exclusive right to commercialize Glybera for LPLD in the European Union and other specified countries, excluding the United States. In July 2013 we received a €2.0 million upfront payment in recognition of our past expenditures incurred in developing the product. In addition, we are eligible to earn up to €42.0 million in commercial milestone payments based on annual sales of Glybera.

We will receive payments for the quantities of Glybera we manufacture and supply to Chiesi, payable in part upon order and in part upon delivery of such product quantities. We will bear the cost of goods sold for the Glybera we deliver, including the royalties and related payments to third parties we must make under the license agreements covering various aspects of the technology underlying the composition and manufacture of Glybera. See "Business—Strategic Collaboration". We estimate that the amount we will retain, net of cost of goods sold, including such third party royalties and related amounts, will be between 20% and 30% of the revenues from sales of Glybera by Chiesi, varying by country of sale. We believe that the amount that we will retain from net sales of Glybera in the European Union will initially be at the lower end of this range and will increase toward the higher end of that range beginning in 2015, upon the expiration of an in-licensed patent on which we pay royalties. In addition, we are required to pay 20% of the gross amount we receive from Chiesi in respect of Glybera product sales to the Dutch government, in repayment of a technical development loan in the outstanding amount of €5.4 million as of September 30, 2013, until the earlier of repayment in full of such amount and 2017, as described below. See "Business—Glybera Commercialization Plan" and "—Intellectual Property—Licenses."

#### ***Hemophilia B agreement***

Under the Hemophilia B agreement, we granted to Chiesi an exclusive license, for the European Union and specified countries other than the United States, to co-develop and exclusively commercialize AMT-060, a gene therapy product for the treatment of hemophilia B. We received a €15.0 million upfront payment under this agreement. Of this amount, €5.0 million related to the future development of our hemophilia B product candidate and €10.0 million related to the use of our manufacturing capacity for our hemophilia B product candidate. In addition, we will share equally with Chiesi specified development expenses attributable to the hemophilia B program according to a defined development plan and budget, including expenses associated with preclinical and clinical studies as well as development and regulatory milestone payments associated with existing in-license agreements. We will receive payments from Chiesi for commercial quantities of our hemophilia B product candidate we manufacture and supply to them, if we receive regulatory approval for such product candidate. We estimate that the amount we would retain, net of cost of goods sold, including third party royalties and related amounts, will be between 25% and 35% of the revenues from sales of such product by Chiesi, varying by country of sale. We and Chiesi have agreed to negotiate a separate supply and distribution agreement in respect of the potential commercialization of our hemophilia B product candidate prior to dosing the first patient in any pivotal study. We are not entitled to any milestone payments under this agreement. See "Business—Strategic Collaboration—Chiesi Farmaceutici."

## **License Agreements**

We have obtained exclusive or non-exclusive rights from third parties under a range of patents and other technology that we are exploiting in Glybera and our development programs. Our agreements with these third parties generally grant us a license to make, use, sell, offer to sell and import products covered by the licensed patent rights in exchange for our payment of some combination of an upfront amount, annual fees, royalties, a portion of amounts we receive from our sub-licensees and payments upon the achievement of specified development, regulatory or commercial milestones. Our potential aggregate financial obligations under these agreements are material. Some of the agreements may also specify the extent of the efforts we must use to develop and commercialize licensed products. See "Business—Intellectual Property—Licenses."

## **Financial Operations Overview**

### **Revenues**

To date, we have not generated any revenues from royalties or product sales. We do not expect to generate royalty or product revenues prior to the commercial launch of Glybera by Chiesi. When and if Chiesi generates commercial sales of Glybera, we will record the gross amounts we receive from Chiesi as product revenues. We will record the related expenses, including third party royalties and related payments, as cost of goods sold.

During the nine months ended September 30, 2013, we recognized collaboration revenues of €1.8 million in respect of development activities that were reimbursable by Chiesi under our co-development agreement for hemophilia B. We expect to continue to recognize such collaboration revenues going forward, in accordance with our contractual agreements.

During the nine months ended September 30, 2013, we also recognized license revenues of €0.2 million. This amount reflects the amortization during the period of the non-refundable upfront payments we received from Chiesi under our collaboration agreements. The balance of €16.8 million of these license revenues will be recognized on a straight-line basis through the remaining period of the intellectual property protection of our manufacturing technologies, which is currently to be until September 2032.

The timing of our operating cash flows may vary from the recognition of the related amounts, as we defer the recognition of some upfront payments, including the upfront payments under our Chiesi agreements, and recognize these as revenue when earned or over a defined period, while we treat other revenue, such as milestone payments or service fees, as earned when received. We expect our revenues to vary from quarter to quarter and year to year, depending upon, among other things, the commercial success of Glybera, our success in obtaining marketing approval for Glybera in the United States and additional countries, the structure and timing of milestone events, the number of milestones achieved, the level of revenues earned for ongoing development efforts, any new collaboration arrangements we may enter into and the terms we are able to negotiate with our collaborators. We currently intend to sell Glybera in the United States, if approved, ourselves, in which case we would recognize revenues in the full amount of the sales price. In addition, because LPLD is an orphan disease and we expect that the number of patients that will be treated with Glybera is relatively small, and because we currently expect that we will receive a one-time payment for a single patient treatment, we anticipate that revenues from Glybera may vary significantly from period to period. Further, because we currently anticipate that LPLD patients will require only a single administration with Glybera, we do not expect to earn recurring revenue from treated patients. We therefore believe that period to period comparisons should not be relied upon as indicative of our future revenues.

**Other Income**

Our other income consists principally of government grants, subsidies and investment credits that support our research efforts in defined research and development projects, which we refer to as grants. These grants generally provide for reimbursement of our approved expenses incurred as defined in various grants. We recognize grants when expenses are incurred in accordance with the terms and conditions of the grant and the collectability of the receivable is reasonably assured. Because we have limited or no control over the timing of receipt of grants, the amount of other income varies from period to period and is not indicative of underlying trends in our operations.

We have received grants from the Dutch government and from the European Union. We have also participated in collaborations and consortia in which our collaborators and fellow consortium members have received grants from governmental authorities, which have enabled us to access preclinical and clinical data while minimizing the expenses we incur.

We have received a research and development subsidy from the Dutch government in the form of reimbursement of payroll taxes related to relevant employees. The amount we receive is tied directly to the number of employees and number of hours devoted to specified research and development programs, and therefore varies directly with the size of our workforce and direction of our research and development programs. We have no obligation to repay these amounts.

Some of the grants we have received are repayable under specified circumstances. In particular, we would be required to repay some grants if we successfully commercialize a supported program within a specified timeframe. None of the grants we have received to date relate to programs that we currently anticipate commercializing, other than the technical development loan in respect of Glybera, described under "Costs of Goods Sold" below. Accordingly, we do not currently expect that we will be required to repay any of these grants.

Other income also includes amounts we receive as payment or reimbursement for expenses of manufacturing and development of AMT-110 under our collaboration agreement with Institut Pasteur.

**Costs of Goods Sold**

Costs of goods sold include the purchase price of raw materials, directly attributable labor costs and directly related charges by third party service providers, and the royalties and other related payments to third parties we must make under the license agreements covering various aspects of the technology underlying the composition and manufacture of Glybera.

We also include in costs of goods sold amounts that we are required to repay to the Dutch government in respect of a technical development loan that we received in the period from 2000 to 2005 to support the early development of Glybera. As of September 30, 2013, the total amount of principal and interest outstanding was €5.4 million. Under the terms of this contingent commitment, we are required to make repayments based on the timing and amount of revenues we receive from product sales of Glybera. In connection with our receipt of upfront payments from Chiesi for the commercialization of Glybera, we repaid €0.8 million of this loan in September 2013, which we recorded as costs of goods sold although no product sales occurred. No further payments will be made until the Company starts the sale of Glybera. We expect to pay to the Dutch government 20% of any gross amounts we receive from Chiesi in connection with sales of Glybera, as and when received, until the earlier of such time as the loan is repaid in full on December 31, 2017. Amounts that remain outstanding as of December 31, 2017, if any, would be forgiven. We have not recorded any liability for these amounts. To the extent we generate further revenues from the sale of Glybera, we will recognize a liability and a corresponding charge to cost of goods sold in future periods.

Should we obtain marketing approval in the United States for Glybera, we expect that our costs of goods sold for sales of Glybera in the United States would be significantly lower than our costs of goods sold for sales of Glybera in the European Union due principally to the existence of lower royalty obligations on United States sales.

### **Research and Development Expenses**

Research and development expenses consist principally of expenses associated with employees, manufacturing facilities, clinical development, collaboration with third parties, license fees, laboratory consumables and depreciation.

During the period from 2006, when we received our first significant venture capital equity investment, to September 30, 2013, we incurred an aggregate of €101.1 million in research and development expenses. We expect that our total research and development expenses in 2013 will be in the range of €12.0 million to €14.0 million. In addition, we began to capitalize our development expenses related to Glybera from March 21, 2013. We capitalized €2.1 million of such expenses in the first nine months of 2013, which we expect to begin amortizing once sales of Glybera commence, over the period through September 2032. We allocate our direct research and development expenses to our various programs on the basis of actual external expenses incurred in respect of each program and our allocation of time spent by our research and development team on each program. We do not allocate our overhead expenses to specific development programs. Our research and development expenses mainly relate to the following key programs:

- *Glybera.* We are undertaking preparations for the EMA-mandated post-approval clinical trial and patient registry. In addition, we are undertaking preparations for the submission of an IND with the FDA in the first half of 2014. We bear all of the costs of this program outside of the territories covered by the Chiesi agreement. Certain costs, including the patient registry for territories covered by the Chiesi agreement, will be shared equally with Chiesi.
- *Hemophilia B.* We plan to initiate a Phase I/II clinical trial of AMT-060 for the treatment of hemophilia B in the second half of 2014 in collaboration with Chiesi. Under our co-development agreement, we and Chiesi will each bear half of the development costs of this program.
- *Acute intermittent porphyria (AIP).* We have incurred costs related to the development and manufacture of clinical supplies of AMT-021 for the treatment of AIP provided to our collaboration partner, Digna Biotech, for its ongoing Phase I clinical trial in this indication.
- *CNS programs.* We have incurred costs related to the development and manufacture of clinical supplies of AMT-110 for the treatment of Sanfilippo B provided to our collaboration partner, Institut Pasteur, for its ongoing Phase I/II clinical trial. We also incur expenses related to the research and preclinical activities related to our other CNS programs.
- *Technology platform development and other research.* We incur significant research and development costs related to our gene delivery and manufacturing technology platform that are applicable across all of our programs, as well as our other research programs, including intellectual property expenses, depreciation expenses and facility costs. These costs are not allocated to specific projects.

The table below sets forth our direct research and development expenses by program for the years ended December 31, 2011 and 2012 and the nine month periods ended September 30, 2012 and 2013:

(€ in thousands, except percentages)	YEAR ENDED DECEMBER 31,			NINE MONTHS ENDED SEPTEMBER 30,		
	2011	2012	CHANGE %	2012	2013	CHANGE %
Glybera program*	4,381	1,055	(76)	651	1,665	156
Hemophilia B program	671	1,131	69	674	1,510	124
AIP program	1,383	1,055	(24)	896	219	(76)
CNS programs	363	922	154	564	804	43
Technology platform development and research programs	8,702	6,068	(30)	2,905	5,658	95
<b>Total</b>	<b>15,500</b>	<b>10,231</b>	<b>(34)</b>	<b>5,690</b>	<b>9,856</b>	<b>73</b>

\* Excludes capitalized development expenses.

Our research and development expenses may vary substantially from period to period based on the timing of our research and development activities, including regulatory approvals and enrollment of patients in clinical trials. We expect that our research and development expenses will increase significantly as we increase our staff, conduct further clinical development of Glybera, advance the research and development of our other product candidates and commence manufacturing at our manufacturing facility in Lexington, Massachusetts. The successful development of our product candidates is highly uncertain. At this time, we cannot reasonably estimate the nature, timing or estimated costs of, or any cash inflows resulting from, the development of any of our product candidates. This is due to numerous risks and uncertainties associated with developing gene therapies, including the uncertainty of:

- the scope, rate of progress and expense of our research and development activities;
- clinical trial and early-stage results;
- the terms and timing of regulatory approvals;
- the expense of filing, prosecuting, defending and enforcing patent claims and other intellectual property rights; and
- our and our collaborators' ability to market, commercialize and achieve market acceptance for Glybera or any other product candidate that we may develop in the future.

A change in the outcome of any of these variables with respect to the development of Glybera or any other product candidate that we may develop could mean a significant change in the expenses and timing associated with the development of Glybera or such product candidate. For example, if the FDA or other regulatory authority were to require us to conduct preclinical and clinical studies for Glybera or any other product candidate beyond those which we currently anticipate will be required for the completion of clinical development, or if we experience significant delays in enrollment in any clinical trials, we could be required to expend significant additional financial resources and time on the completion of the clinical development. See "Risk Factors—Risks Related to the Development of Our Product Candidates" and "—Risks Related to the Regulatory Approval of Our Product Candidates".

We have incurred significant expenses in the development of Glybera. Under applicable accounting principles, we capitalize development expenses upon receipt of marketing approval for a product candidate, provided that we have the technical, scientific and financial resources to complete the development and commercialization of the program. We received marketing approval from the European Commission for Glybera for a subset of LPLD patients in October 2012. Because we did not have sufficient financial resources at that time to complete the development of Glybera, including the post-approval activities required by the EMA prior to commercial launch, we did not capitalize the development expenses related to

Glybera during the year ended December 31, 2012. Following our receipt of an additional €10.0 million in convertible debt financing in the first quarter of 2013, we determined that we had sufficient financial resources to complete these post-approval activities, and accordingly began to capitalize the related development expenses in the first quarter of 2013.

Over the period through 2016, we anticipate that we will incur external expenses related to the further development of Glybera, including implementation of the patient registry, initiation and conduct of the post-approval clinical trial and potential additional development work to obtain FDA approval, of approximately €7.0 million; in addition, we will incur significant related employee expenses. See "Risk Factors—Risks Related to the Development of Our Product Candidates" and "—Risks Related to the Regulatory Approval of Our Product Candidates."

### ***Selling, General and Administrative Expenses***

Our selling, general and administrative expenses have consisted to date principally of employee, office, consultancy and other administrative expenses. We expect that our selling, general and administrative expenses will increase significantly in the future as our business expands and we add personnel, particularly in our medical affairs, commercial, quality control, finance and compliance groups, and as we commence manufacturing operations in our facility in Lexington, Massachusetts. We also expect to incur additional expenses associated with operating as a public company, including expenses for additional personnel, additional legal, accounting and audit fees, directors' and officers' liability insurance premiums and expenses related to investor relations. In future periods, we will include in selling, general and administrative expenses our sales expenses related to the commercialization of Glybera in the European Union, including our market access and medical affairs efforts, as well as the costs related to the sales and marketing efforts we intend to undertake in the United States in advance of potential marketing approval for Glybera from the FDA.

### ***Other Losses—Net***

Other losses—net consists of foreign exchanges losses that do not relate to borrowings. We are exposed to foreign exchange risk arising from various currency exposures, primarily with respect to the U.S. dollar and, to a lesser extent, the British pound, as we acquire certain materials and pay for certain licenses and other services in these two currencies. We have not established any formal practice to manage the foreign exchange risk against our functional currency.

### ***Finance Income***

Our finance income consists of interest income earned on our cash and cash equivalents and gains on our derivative instruments, described below. We deposit our cash and cash equivalents primarily in savings and deposit accounts with original maturities of three months or less. Savings and deposit accounts have historically generated only minimal interest income.

We have entered into various financing arrangements with our investors, including convertible notes issued in 2009, 2012 and 2013, all of which were converted into ordinary shares in July 2013. See "Related Party Transactions" for further detail. Each of the convertible notes consists of a debt element and an embedded financial derivative element. Derivatives are initially recognized at fair value on the date a derivative contract is entered into and are subsequently measured at fair value through profit and loss. The resulting gain is recognized in the consolidated income statement and accounted for as finance income.

### ***Finance Expense***

Finance expense consists primarily of interest due on our convertible notes, losses on the fair value measurements of our derivative instruments, and, to a lesser extent, the interest component of finance leases.



## Results of Operations

### Overview

Our results of operations in the periods under review were significantly affected by the corporate reorganization and strategic restructuring, and related contraction of our research and development and other activities, that we initiated at the end of 2011 in order to conserve resources and improve our financial position following the initial rejection of the application for marketing approval for Glybera in the European Union. Following the approval of Glybera in the European Union in October 2012 and additional investment received in the first quarter of 2012, we began to significantly expand our operations.

### Comparison of the nine months ended September 30, 2012 and 2013

(€ in thousands)	NINE MONTHS ENDED SEPTEMBER 30,		
	2012	2013	CHANGE
<b>Revenues:</b>			
License revenues	€ —	€ 220	—%
Collaboration revenues	—	1,831	—
<b>Total revenues</b>	—	<b>2,051</b>	—
Cost of goods sold	—	(800)	—
<b>Gross profit</b>	—	<b>1,251</b>	—
Other income	315	686	118
<b>Expenses:</b>			
Research and development expenses	(5,690)	(9,856)	73
Selling, general and administrative expenses	(4,438)	(7,612)	72
Other gains/losses—net	(82)	(269)	228
<b>Operating result</b>	<b>(9,895)</b>	<b>(15,800)</b>	<b>60</b>
Finance income	16	48	200
Finance expense	(545)	(4,676)	758
<b>Net loss</b>	<b>(10,424)</b>	<b>(20,428)</b>	<b>96</b>

### Revenues

License revenues of €0.2 million in the nine months ended September 30, 2013, related to the amortization of the up front payment received from Chiesi in July 2013.

Collaboration revenues of €1.8 million in the nine months ended September 30, 2013 consisted mainly of reimbursements of covered expenses by Chiesi under our co-development agreement for hemophilia B, together with revenue from Institut Pasteur relating to our collaboration with them on Sanfilippo B. We had no revenues in the nine months ended September 30, 2012.

### Cost of Goods Sold

Cost of goods sold of €0.8 million in the nine months ended September 30, 2013 consisted of the recognition of a repayment obligation to the Dutch government with respect to a portion of a technical development loan. This repayment obligation was triggered by our entitlement to receive during the second quarter 2013 a €2.0 million upfront payment from Chiesi in relation to our Glybera program. We had no cost of goods sold in the first nine months of 2012.

**Other Income**

Other income for the nine months ended September 30, 2013 was €0.69 million, a 118% increase from the €0.31 million recognized for the nine months ended September 30, 2012. This increase principally reflected an increase of €0.15 million in the amount of reimbursement of payroll taxes received from the Dutch government as a result of higher headcount in 2013, reflecting the lower staff numbers in the prior period following our strategic restructuring at the end of 2011. The remainder of the increase reflected the receipt of €0.23 million of grants to support research projects.

**Research and Development Expenses**

Research and development expenses for the nine months ended September 30, 2013 were €9.9 million, a 73% increase from the €5.7 million incurred for the nine months ended September 30, 2012. This increase reflected the expansion of our research and development activities to support the planned commercial launch of Glybera as well as the further development of Glybera and our pipeline product candidates. Following our receipt of additional convertible loan and debt funding in the first nine months of 2013, we increased the level of research and development expenditures compared with the relatively low level of expenditure during 2012 attributable to our strategic restructuring at the end of 2011.

Glybera-related raw materials that cannot be used for commercial purposes are expensed; Glybera-related materials, including raw materials, work-in-progress and finished goods, that are expected to be used for commercial purposes are recorded as inventory on the balance sheet and are not accounted for within research and development expenses.

**Selling, General and Administrative Expenses**

Selling, general and administrative expenses for the nine months ended September 30, 2013 were €7.6 million, a 72% increase from the €4.4 million incurred for the nine months ended September 30, 2012. This increase resulted principally from our increased headcount in 2013 as we continued to ramp up our operations following our strategic restructuring at the end of 2011 and increased commercial, legal and other advisory fees.

**Other losses—Net**

Other losses—net for the nine months ended September 30, 2013 were a loss of €0.27 million, a 228% increase from the loss of €0.08 million for the nine months ended September 30, 2012, and related to foreign exchange impacts. This increase reflects changes in the exchange rate between the euro and the U.S. dollar.

**Finance Income**

Finance income for the nine months ended September 30, 2013 was €0.05 million, a 200% increase from the €0.01 million for the nine months ended September 30, 2012. This reflects our low average cash balances and low interest rates in both periods.

**Finance Expense**

Finance expense for the nine months ended September 30, 2013 was €4.7 million, compared with €0.55 million for the nine months ended September 30, 2012. This increase primarily related to the revaluation of the embedded derivatives related to the convertible loans and the venture debt, which totaled €3.7 million during the nine months ended September 30, 2013.

**Comparison of the years ended December 31, 2011 and 2012**

(€ in thousands)	YEAR ENDED DECEMBER 31,		
	2011	2012	CHANGE %
<b>Revenues:</b>	€ —	€ —	—
License revenues	—	—	—
Collaboration revenues	—	—	—
<b>Total revenues</b>	—	—	—
Cost of goods sold	—	—	—
<b>Gross loss</b>	—	—	—
<b>Other income</b>	2,192	649	(70)
Research and development expenses	(15,500)	(10,231)	(34)
Selling, general and administrative expenses	(3,807)	(4,564)	20
Other losses, net	(26)	(45)	73
Finance income	277	22	(92)
Finance expense	(436)	(547)	25
<b>Net loss</b>	€ (17,300)	€ (14,716)	(15)

**Other Income**

Other income for the year ended December 31, 2012 was €0.6 million, a 70% decrease from the €2.2 million recognized for the year ended December 31, 2011. The higher amounts in 2011 reflected a grant in the amount of €1.0 million accounted for in that period from the European Union through our collaborator in connection with our AIP program, as well as €0.8 million from our collaborator Institut Pasteur related to the supply by us of material for use in the Sanfilippo B program. The reduction in the amount of Other Income in 2012 reflects the variable nature of payments receivable under these arrangements.

**Research and Development Expenses**

Research and development expenses for 2012 were €10.2 million, a 34% decrease from the €15.5 million incurred for the year ended December 31, 2011. The decrease reflected the strategic restructuring and related reduction in our workforce we undertook at the end of 2011. Following the reduction in staff, we also reduced our overall level of activity. Furthermore, during the first half of 2012, we focused on our early-stage programs, which generally require less investment than more advanced programs.

**Selling, General and Administrative Expenses**

Selling, general and administrative expenses for 2012 were €4.6 million, a 20% increase from the €3.8 million incurred for the year ended December 31, 2011. This increase reflected principally increased legal and other advisory costs incurred in 2012 in connection with our corporate reorganization, described above, and to a lesser extent expanded business development activities in 2012.

**Other Losses—Net**

Other losses—net were not material in either 2012 or 2011.

**Finance Income**

Finance income was nominal in 2012, compared with €0.3 million in 2011, reflecting the low average cash balances during 2012 during a period when interest rates available on cash deposits were low.

**Finance Expense**

Finance expense remained relatively stable at €0.5 million in 2012 compared with €0.4 million in 2011, principally representing interest due on convertible loans in 2011, and the charge on the movement in the value of the derivative element of the convertible loan, which was converted on our restructuring in April 2012.

**Liquidity and Capital Resources**

In our early years we received funding and subsidized rent from the AMC, government grants, income for cGMP contract manufacturing of biologics for third parties, and small amounts of equity financing. Since our first institutional venture capital financing in 2006, we have funded our operations primarily through private and public placements of equity securities, and convertible and other debt securities, in the aggregate amount of €137.5 million (\$179.9 million). During this period, we have also received total other income, consisting principally of government grants and subsidies, of €5.9 million (\$7.7 million), and total nonrefundable collaboration funding of €17.0 million, and \$10.0 million (€7.5 million) in venture debt financing.

We had a net loss of €20.4 million in the first nine months of 2013, €14.7 million in full year 2012 and €17.3 million in full year 2011. As of September 30, 2013, we had an accumulated deficit of €138 million.

**Cash flows**

Our cash and cash equivalents as of September 30, 2013 were €31.4 million. The table below summarizes our consolidated cash flow data for the years ended December 31, 2011 and 2012 and each of the unaudited nine-month periods ended September 30, 2012 and 2013:

(€ in thousands)	YEAR ENDED DECEMBER 31,		NINE MONTHS ENDED SEPTEMBER 30,	
	2011	2012	2012	2013
Net cash (used in)/generated by operating activities	(16,705)	(11,277)	(8,579)	1,674
Net cash used in investing activities	(162)	(832)	(427)	(4,179)
Net cash generated from financing activities	108	11,272	9,433	33,663

**Net Cash (Used in)/Generated by Operating Activities**

Net cash generated by operating activities was €1.7 million in the nine months ended September 30, 2013, a 120% decrease from net cash used in operating activities of €8.6 million in the nine months ended September 30, 2012. The change reflected the receipt of the upfront payment under our collaboration agreements with Chiesi.

Net cash used in operating activities was €11.3 million in 2012, a 33% decrease from €16.7 million in 2011. In 2012 the Result before corporate income tax was €14.7 million, a decrease of €2.6 million in the net loss compared to 2011. A number of adjustments are then made to these losses to derive the net cash used in operating activities. In 2012 the net impact of these adjustments, which included adjustments for share based compensation of €1.8 million (2011: €0.9 million) and changes in trade and other payables of €0.2 million (2011: decrease of €1.0 million), resulted in an overall reduction in the actual cash flows used in operations.

### Net Cash Used in Investing Activities

Net cash used in investing activities was €4.2 million in the nine months ended September 30, 2013, compared with net cash used in investing activities of €0.43 million in the nine months ended September 30, 2012. The increase reflected the capitalization of €2.1 million of Glybera development expenses beginning in March 2013.

Net cash used in investing activities was €0.8 million in 2012, an increase of 414% from €0.2 million in 2011. This increase was due to purchases of intangible assets and, to a lesser extent, purchases of property, plant and equipment.

### Net Cash Generated from Financing Activities

Net cash generated from financing activities was €33.7 million in the nine months ended September 30, 2013, compared with net cash generated from financing activities of €9.4 million in the nine months ended September 30, 2012. The increase reflected the receipt of €12.0 million in funding from the issuances of convertible notes (all of which were fully converted in the period), \$10.0 million in funding from a venture loan and the receipt of the €14.0 equity investment from Chiesi during the nine months ended September 30, 2013.

Net cash generated from financing activities was €11.3 million in 2012, compared with €108,000 in 2011. The increase reflected our private placements of convertible notes and equity securities in 2012 in connection with and following our corporate reorganization.

### Cash and Funding Sources

The table below summarizes our sources of financing for the years ended December 31, 2011 and 2012 and the nine months ended September 30, 2013.

(€ in thousands)	EQUITY CAPITAL <sup>(1)</sup>	CONVERTIBLE NOTES	OTHER DEBT	TOTAL
Nine months ended September 30, 2013	14,278	11,999	7,492	33,769
Year ended December 31, 2012	9,774	1,498	—	11,272
Year ended December 31, 2011	108	—	—	108
<b>Total</b>	<b>24,160</b>	<b>13,497</b>	<b>7,492</b>	<b>45,149</b>

<sup>(1)</sup> Excludes shares issued upon conversion of convertible notes.

Our sources of financing in the nine months ended September 30, 2013 were:

- the issuance and sale of 453,738 our class B ordinary shares to our employees for gross proceeds of €0.3 million;
- the issuance and sale of €12.0 million of our convertible notes;
- a venture loan in the principal amount of \$10.0 million from Hercules Technology Growth Capital, or Hercules, pursuant to a loan agreement dated June 14, 2013, or the Hercules Agreement; and
- the acquisition of 6,681,378 ordinary shares by Chiesi for €14.0 million.

As of September 30, 2013, we had debt of €7.3 million, which consisted solely of amounts outstanding under the Hercules Agreement.

## **Funding Requirements**

We believe that the net proceeds of this offering, together with our existing cash and cash equivalents, will enable us to fund our operating expenses, including our debt repayment obligations as they become due, and capital expenditure requirements, including the build-out of our Lexington, Massachusetts facility, for at least the next months. See "Use of Proceeds." We have based this estimate on assumptions that may prove to be incorrect, and we could use our capital resources earlier than we currently expect. Our future capital requirements will depend on many factors, including:

- the commercial success of Glybera, including the timing and amount of revenues generated, as well as our cost of goods sold;
- our collaboration agreements remaining in effect and our ability to obtain research and development funding and achieve milestones under these agreements;
- the progress and results of our current and planned clinical trials, including for Glybera and those of our collaborators;
- the scope, progress, results and costs of preclinical development, laboratory testing and clinical trials for our additional product candidates;
- the number and development requirements of other product candidates that we pursue;
- the cost, timing and outcome of regulatory review of our product candidates, particularly for approval of Glybera in the United States;
- the cost and timing of future commercialization activities by us or our collaborators, including product manufacturing, marketing, sales and distribution, for Glybera and any of our product candidates for which we receive marketing approval in the future;
- the amount and timing of revenue, if any, we receive from commercial sales of any product candidates for which we receive marketing approval in the future;
- the costs and timing of preparing, filing and prosecuting patent applications, maintaining and enforcing our intellectual property rights and defending any intellectual property-related claims;
- the extent to which we acquire or in-license other products or technologies; and
- the cost and progress of the build-out of our Lexington, Massachusetts manufacturing facility.

We have no committed sources of additional financing, other than our collaboration agreements with Chiesi. Until such time, if ever, as we can generate substantial product revenues from sales of Glybera by Chiesi or otherwise, we expect to finance our cash needs through a combination of equity offerings, debt financings, collaborations, strategic alliances and marketing, distribution and licensing arrangements. To the extent that we raise additional capital through the sale of equity or convertible debt securities, your ownership interest will be diluted, and the terms of these securities may include liquidation or other preferences that adversely affect your rights as an ordinary shareholder. We are subject to covenants under our existing Loan and Security Agreement with Hercules, and may become subject to covenants under any future indebtedness, that could limit our ability to take specific actions, such as incurring additional debt, making capital expenditures or declaring dividends, which could adversely impact our ability to conduct our business. In addition, our pledge of assets as collateral to secure our obligations under our venture loan from Hercules may limit our ability to obtain debt financing. If we raise additional funds through collaborations, strategic alliances or marketing, distribution or licensing arrangements with third parties, we may have to relinquish valuable rights to our technologies, future revenue streams or product candidates or grant licenses on terms that may not be favorable to us. If we are unable to raise additional funds through equity or debt financings when needed, we may be required to delay, limit, reduce or terminate our product development or future commercialization efforts or grant rights to develop and market product candidates that we would otherwise prefer to develop and market ourselves.

For more information as to the risks associated with our future funding needs, see "Risk Factors—Risks Related to Our Financial Position and Need for Additional Capital".

## Capital Expenditures

The following table sets forth our capital expenditures for the years ended December 31, 2011 and 2012, and for the nine months ended September 30, 2013 and 2012.

(€ in thousands)	YEAR ENDED DECEMBER 31,		NINE MONTHS ENDED SEPTEMBER 30,	
	2011	2012	2012	2013
Investments in property, plant and equipment	200	392	149	566
Investments in intangible assets	109	553	386	3,647
<b>Total</b>	<b>309</b>	<b>945</b>	<b>535</b>	<b>4,213</b>

We are currently building out a 53,000 square foot leased manufacturing facility in Lexington, Massachusetts. We anticipate that the total construction costs will amount to approximately \$16.0 million (€11.5 million), of which the landlord is obligated to pay \$7.2 million (€5.5 million) in landlord improvements. In addition, we anticipate the total investment in property, plant and equipment to be approximately \$6.0 million (€4.6 million). As of September 30, 2013, we had incurred expenses of \$0.12 million (€0.09 million) and had contractual commitments of a further \$0.2 million (€0.15 million). In addition, we provided a landlord deposit of \$1.2 million (€0.92 million). We anticipate that we will have paid the full amount of these build-out costs by the end of the second quarter of 2014.

In October 2013, we entered into additional preconstruction commitments to complete the design and planning work, to acquire the building permit and to order certain materials and equipment that require a longer lead time. The combined sum of these additional commitments entered into amounted to \$4.9 million, which is expected to be paid partially in the fourth quarter of 2013 and partially in early 2014.

We also anticipate that we will incur additional capital expenditures related to our planned expansion of our facility in Amsterdam.

## Contractual Obligations and Commitments

The table below sets forth our contractual obligations and commercial commitments as of September 30, 2013 that are expected to have an impact on liquidity and cash flow in future periods.

(€ in thousands)	PAYMENTS DUE BY PERIOD				
	LESS THAN 1 YEAR	BETWEEN 1 AND 2 YEARS	BETWEEN 2 AND 5 YEARS	MORE THAN 5 YEARS	TOTAL
License maintenance obligations	—	—	—	—	—
Research and development related contractual obligations	298	—	—	—	298
Debt obligations	1,165	2,690	4,601	—	8,456
Operating lease obligations	542	542	678	—	1,762
Finance lease obligations	153	165	177	—	495
<b>Total</b>	<b>2,158</b>	<b>3,397</b>	<b>5,456</b>	<b>—</b>	<b>11,011</b>

The table above does not include:

- Payments we may be obligated to make under our license or collaboration agreements, other than fixed periodic maintenance costs. Such additional payment obligations may be material. See "—Collaboration and License Agreements" and "Business—Intellectual Property—Licenses".
- Our obligations to repay the Dutch technical development loan described above.
- Payments in relation to the lease of the Lexington facility. These payments begin 7 months after the landlord completed the required initial works to the building, which occurred on November 5, 2013. As of September 30, 2013, we considered the Lexington lease obligations a contingency and not yet a commitment as the landlord had remaining pre-existing obligations prior to the lease commitment. From November 5, 2013, we will account for these lease commitments as accruals under IFRS and will recognize the benefit of the 7 month rent-free period over the duration of the lease.
- Payments in relation to the pre-construction commitments described above.

### ***Hercules Loan and Security Agreement***

We are party to a Loan and Security Agreement entered into with Hercules on June 13, 2013. Under the Loan and Security Agreement, we borrowed \$10.0 million from Hercules, bearing interest at a variable rate of the greater of 11.85% or an amount equal to 11.85% plus the prime rate of interest minus 3.25%. We are required to pay only interest in monthly payments until October 2014. From October 2014, we will be required to make monthly payments of interest and principal in the amount of \$387,000 (€295,872). The loan matures on October 1, 2016, when we will be required to make a final payment of \$2.6 million (€2.1 million). The loan and security agreement also provides for payment of a maturity charge, the amount of which was reduced in exchange for the issuance to Hercules, on September 24, 2013, of 185,873 warrants, at an exercise price of €2.02 per share.

We have pledged substantially all of our assets as collateral to the Hercules loan, by means of a first ranking right of pledge. The Loan and Security Agreement contains covenants that restrict our ability to, among other things, incur future indebtedness and obtain additional financing, to make investments in securities or in other companies, to transfer our assets, to perform certain corporate changes, to make loans to employees, officers and directors, and to make dividend payments and other distributions. Further, we are required to keep a minimum cash balance deposited in bank accounts in the United States, equivalent to the lesser of the outstanding balance of principal due and 50% of our worldwide cash reserves. The Loan and Security Agreement contains default provisions that include the occurrence of a material adverse effect, as defined therein, that would entitle Hercules to declare all principal, interest and other amounts owed by us immediately due and payable.

### ***Off-Balance Sheet Arrangements***

Over the period from October 1, 2000 through May 31, 2005, we received a grant called a "Technisch ontwikkelingskrediet," or technical development loan, from the Dutch government. We received grants totaling €3.6 million during the grant period. The grant amount bears interest of 5.7% per year and includes a repayment clause in the event we generate revenues from Glybera, during the period from January 1, 2008 through December 31, 2017, based upon a percentage of revenues which are derived from the sale of Glybera, if any. If future amounts received are not sufficient to repay the grant on or prior to December 3, 2017, or if there are no revenues generated from Glybera, the remaining balance will be forgiven. The amount of this contingent commitment as of September 30, 2013 totaled €5.4 million, comprising the original grant together with accrued interest. We have not recorded any liability to repay amounts in respect of this contingent commitment. Further amounts may be recognized once revenues related to produce sales at Glybera commence.

As of the date of this prospectus, and during the periods presented in this prospectus, we did not have any other off-balance sheet arrangements.



## **Quantitative and Qualitative Disclosures about Market Risk**

We are exposed to a variety of financial risks, including market risk (including currency risk, price risk and cash flow and fair value interest rate risk), credit risk and liquidity risk. Our overall risk management program focuses on preservation of capital and the unpredictability of financial markets and has sought to minimize potential adverse effects on our financial performance and position.

### ***Market Risk***

We are exposed to foreign exchange risk arising from various currency exposures, primarily with respect to the U.S. dollar, particularly as we expand our operations in the United States and build-out our manufacturing facility in Lexington, Massachusetts. We have not established any formal practice to manage the foreign exchange risk against our functional currency. As of September 30, 2013, we had no significant outstanding receivables or payables in currencies other than euros, other than our loan from Hercules, which was received and is repayable in U.S. dollars. Subsequent to September 30, 2013, we have incurred obligations in U.S. dollars in respect of our manufacturing facility in Lexington, Massachusetts, as described above. Foreign exchange rate movements had no material effect during the periods under review.

Our interest rate risk arises from short and long-term borrowings. As of December 31, 2012, we had no borrowings with variable rates and we were not exposed to cash flow interest rate risk. In June 2013, we entered into the Hercules Agreement under which our borrowings bear interest at a variable rate. Borrowings issued at fixed rates expose us to fair value interest rate risk.

As of December 31, 2012, we had neither significant long-term interest-bearing assets nor significant long-term interest bearing liabilities other than our convertible notes, which were subsequently converted into 6,681,678 of our class A ordinary shares on July 26, 2013. As of September 30, 2013, the loans issued under the Hercules Agreement bore interest at the rate of the greater of 11.85% and an amount equal to 11.85% plus the prime rate of interest minus 3.25%.

### ***Credit Risk***

We have a limited group of material external counterparties, of which the most significant is Chiesi. Over the coming years, funding under our collaboration and co-development agreements with Chiesi, including milestone payments, collaboration revenues and reimbursable research expenses, remains critical for our product development programs and represents our principal credit risk.

Our cash and cash equivalents are invested primarily in savings and deposit accounts with original maturities of three months or less. Savings and deposit accounts generate a small amount of interest income. For banks and financial institutions, we accept only independently rated parties with a minimum rating of 'A-'.

### ***Liquidity Risk***

We believe that the net proceeds of this offering, together with our existing cash and cash equivalents and anticipated payments under our agreements with Chiesi will enable us to fund our operating expenses and capital expenditure requirements for at least the next                      months. See "Use of Proceeds."

## **Internal Control Over Financial Reporting**

In connection with the preparation and external audit of our consolidated financial statements as of and for the year ended December 31, 2012 and the review of our consolidated financial statements as of and for the nine months ended September 30, 2013, we and our auditors, an independent registered public accounting firm, noted three material weaknesses in our internal control over financial reporting. The material weaknesses identified were:

- a lack of accounting resources to fulfilling IFRS and SEC reporting requirements,

- a lack of comprehensive IFRS accounting policies and financial reporting procedures; and
- a lack of segregation of duties given the size of our finance and accounting team.

We have implemented and are continuing to implement various measures to address the material weaknesses identified; these measures are outlined below.

Neither we nor our independent registered public accounting firm undertook a comprehensive assessment of our internal control for purposes of identifying and reporting material weaknesses, significant deficiencies and control deficiencies in our internal control over financial reporting as we will be required to do once we become a public company. We believe it is possible that, had we performed a formal assessment of our internal control over financial reporting or had our independent registered public accounting firm performed an audit of our internal control over financial reporting, additional control deficiencies may have been identified.

We have begun the process of evaluating our internal control over financial reporting, although we may not complete our review until after this offering is completed. We have also taken several remedial actions to address the material weaknesses that have been identified. To this end, we have hired additional staff for the finance department who have external reporting and IFRS experience, and experience with establishing appropriate financial reporting policies. Moreover, we have engaged a team of external consultants to assist us to improve our corporate governance and internal control procedures and help us design and implement a structured control environment for complying with the Sarbanes-Oxley Act of 2002, and we have devoted significant efforts to remedy any deficiencies or control gaps identified in the process. We expect to complete the measures above as soon as practicable and we will continue to implement measures to remedy our internal control deficiencies in order to meet the deadline imposed under Section 404 of the Sarbanes-Oxley Act. However, the implementation of these measures may not fully address the existing material weaknesses in our internal control over financial reporting, and we cannot yet conclude that they have been fully remedied.

The process of designing and implementing an effective financial reporting system is a continuous effort that requires us to anticipate and react to changes in our business and the economic and regulatory environments and to expend significant resources to maintain a financial reporting system that is adequate to satisfy our reporting obligations. See "Risk Factors—Risks Related to this Offering and Ownership of our Ordinary Shares—If we fail to implement and maintain an effective system of internal control, we may be unable to accurately report our results of operations or prevent fraud or fail to meet our reporting obligations, and investor confidence and the market price of our common shares may be materially and adversely affected."

#### **Critical Accounting Policies and Significant Judgments and Estimates**

Our management's discussion and analysis of our financial condition and results of operations is based on our consolidated financial statements, which we have prepared in accordance with IFRS as issued by the IASB. The preparation of these financial statements requires us to make estimates and assumptions that affect the reported amounts of assets and liabilities and the disclosure of contingent assets and liabilities at the date of the financial statements, as well as the reported revenues and expenses during the reporting periods. Actual results may differ from these estimates under different assumptions or conditions.

Our significant accounting policies are more fully described in the notes to our consolidated financial statements appearing elsewhere in this prospectus. We believe that the following accounting policies involve the most significant judgments and estimates by management and are the most critical to aid you in fully understanding and evaluating our financial condition and results of operations.

## **Revenue Recognition**

We have not generated any revenues from royalties or product sales for any periods covered by the financial statements included in this prospectus.

During 2013, we received upfront payments in connection with our Glybera commercialization agreement and hemophilia B co-development agreement, each with Chiesi. Revenues from such non-refundable, up-front payments are initially reported as deferred revenues on the consolidated balance sheet and are recognized in revenues on the income statement as earned over the period of the development, commercialization, collaboration or manufacturing obligation.

We also generate revenues from collaborative research and development arrangements. Such agreements may consist of multiple elements and provide for varying consideration terms, such as up-front, milestone and similar payments, which require significant analysis by management in order to determine the appropriate method of revenue recognition.

Where such arrangements can be divided into separate units of accounting (each unit constituting a separate earnings process), the arrangement consideration is allocated to the different units based on their relative fair values and recognized over the respective performance period. Where the arrangement cannot be divided into separate units, the individual deliverables are combined as a single unit of accounting and the total arrangement consideration is recognized over the estimated collaboration period. This analysis requires considerable estimates and judgments to be made by us, including estimates of the relative fair values of the various elements included in such agreements and the estimated length of the respective performance periods.

Non-refundable upfront payments received from Chiesi related to licenses and reimbursement of past development costs for Glybera and our hemophilia B program. We have concluded that the elements of the payments are linked in such a way that the commercial effect cannot be understood without reference to the series of transactions as a whole. Therefore the individual performance obligations have been treated as a single unit of accounting and the total arrangement consideration is recognized over the estimated life of the agreements under which the continuing performance obligations exist.

## **Research and Development Expenses**

We recognize research expenses as incurred. We recognize expenses incurred on development projects as intangible assets as of the date that it can be established that it is probable that future economic benefits that are attributable to the asset will flow to us, considering the development projects' commercial and technological feasibility, generally when we receive regulatory approval for commercial sale, and when expenses can be measured reliably. Given the current stage of the development of our products and product candidates, we did not capitalize any development expenditures prior to 2013. As noted above, we incurred significant expenses in the development of Glybera. We received marketing approval from the European Commission for Glybera for a subset of LPLD patients in October 2012. Because we did not have sufficient financial resources at that time to complete the development of Glybera, including the post-approval activities required by the EMA prior to commercial launch, however, we did not capitalize the development expenses related to Glybera during the year ended December 31, 2012. Following our receipt of an additional €10.0 million in convertible debt financing in the first quarter of 2013, we determined that we had sufficient financial resources to complete these post-approval activities, and accordingly began to capitalize the related development expenses from March 21, 2013. Glybera-related raw materials that cannot be used for commercial purposes are expensed; Glybera-related materials, including raw materials, work-in-progress and finished goods, that are expected to be used for commercial purposes are recorded as inventory on the balance sheet and are not accounted for within research and development expenses.

As of each balance sheet date, we estimate the level of service performed by our vendors or other counterparties and the associated costs incurred for the services performed. As part of the process of

preparing our financial statements we are required to estimate our accrued expenses. This process involves reviewing quotations and contracts, identifying services that have been performed on our behalf, estimating the level of service performed and the associated costs incurred for the service when it has not yet been invoiced or otherwise notified of the actual costs. The majority of our service providers invoice us monthly in arrears for services performed or when contractual milestones are met. We make estimates of our accrued expenses as of each balance sheet date in our financial statements based on facts and circumstances known to us at that time. We periodically confirm the accuracy of our estimates with the service providers and make adjustments if necessary. The significant estimates in our accrued research and development expenses are related to fees paid to clinical research organizations, or CROs, in connection with research and development activities for which we have not yet been invoiced. We base our expenses related to CROs on our estimates of the services received and efforts expended pursuant to quotes and contracts with CROs that conduct research and development on our behalf.

The financial terms of these agreements are subject to negotiation, vary from contract to contract and may result in uneven payment flows. There may be instances in which payments made to vendors and other counterparties will exceed the level of services provided and result in a prepayment of the research and development expenses. In accruing service fees, we estimate the time period over which services will be performed and the level of effort to be expended in each period. If the actual timing of the performance of services or the level of effort varies from our estimate, we adjust the accrual or prepayment expense accordingly. Although we do not expect our estimates to be materially different from amounts actually incurred, our understanding of the status and timing of services performed relative to the actual status and timing of services performed may vary and could result in reporting amounts that are too high or too low in any particular period.

### ***Corporate and Other Taxes***

We are subject to corporate taxes in the Netherlands and the United States. Significant judgment is required in determining the use of net operating loss carry forwards and taxation of upfront and milestone payments for corporate tax purposes. There are many transactions and calculations for which the ultimate tax determination is uncertain. Where the final tax outcome of these matters is different from the amounts that were initially recorded, such differences will impact the current and deferred corporate tax assets and liabilities in the period in which such determination is made.

We did not recognize any taxes or income during the periods covered by financial statements contained in this prospectus, since we are in a loss making position and have a history of losses. As of December 31, 2012, the total amount of tax losses carried forward was €106.3 million.

We have a history of tax losses, and therefore only recognize deferred tax assets arising from unused tax losses or tax credits only to the extent that the relevant consolidated Dutch entity has sufficient taxable temporary differences or there is convincing other evidence that sufficient taxable profit will be available against which the unused tax losses or unused tax credits can be utilized by the consolidated Dutch entities. Management believes that sufficient convincing other evidence is not currently available and therefore we have not recorded a deferred tax asset in the financial statements contained in this prospectus. Tax losses in the Netherlands may be carried forward for nine years.

### ***Impairments of Assets***

Assets that are subject to amortization are reviewed for impairment whenever events or changes in circumstances indicate that the carrying amount may not be recoverable. In the nine months ended September 30, 2013 and year ended December 31, 2012, we have reviewed the carrying amount of these assets and determined that no adjustments to carrying values were required.

In the year ended December 31, 2011, we recorded an impairment charge of €0.3 million in respect of the termination of a research license under which uniQure had made an initial payment of €0.3 million.

We test assets that are not subject to amortization annually for impairment. For the purpose of assessing impairment, we group assets at the lowest levels for which there are separately identifiable cash flows (cash-generating units). We currently use all material assets in the development of certain gene therapy products. Therefore, our management regularly reviews all activities of our group as a single component and one cash-generating unit. Although we are not currently selling any products, our collaborator, Chiesi, is preparing the commercial launch of Glybera in the European Union. Our future revenues from product sales, if any, will depend on the success of Chiesi's commercialization efforts in the European Union and our success in obtaining marketing authorization for Glybera and any other product candidates in additional countries.

We have determined that no impairment should be recorded during the year ended December 31, 2012 or the first nine months of 2013. Based on our expectations of revenues and gross margin from anticipated sales of Glybera by Chiesi, we have determined that no impairment charge in respect of intangible assets relating to Glybera is necessary. These expectations are based principally on our estimate of the market size for Glybera and the gross margin that we expect to realize.

### ***Compound Financial Instruments***

We classify a financial instrument or its component parts on initial recognition as a financial liability, a financial asset or an equity instrument in accordance with the substance of the contractual arrangement and the definitions of a financial liability, a financial asset and an equity instrument. We have analyzed the convertible loans we issued in 2012 and 2013 and the venture debt financing received from Hercules in 2013, and concluded that both instruments were composed of a loan component and an embedded financial derivative component, which qualified as financing liabilities. We estimated the fair value of these liabilities using option pricing models and assumptions that are based on the individual characteristics of the instruments on the valuation date.

### ***Share-Based Compensation***

We issue share-based compensation awards, in the form of options to purchase ordinary shares, to certain of our employees, supervisory board members and consultants. We measure share-based compensation expense related to these awards by reference to the estimated fair value of the award at the date of grant. The total amount of the awards is expensed over the estimated vesting period. We have used the Black-Scholes option pricing model to determine the fair value of option awards, which requires the input of various assumptions that require management to apply judgment and make assumptions and estimates, including:

- the expected life of the option award, which we calculate using the simplified method as we have insufficient historical information regarding our share options to provide a basis for estimate;
- the expected volatility of the underlying ordinary shares, which we estimate based on the historical volatility of a peer group of comparable publicly traded companies with product candidates in similar stages of development; and
- historically, the fair value of our ordinary shares determined on the date of grant.

At each balance sheet date, we revise our estimates of the number of options that are expected to become exercisable. We recognize the impact of the revision of original estimates, if any, in the statement of comprehensive income and a corresponding adjustment to equity. We expect all vested options to be exercised over the remainder of their contractual life. We consider the expected life of the options to be in line with the average remaining term of the options post vesting.

Prior to our acquisition of the AMT business on April 5, 2012, AMT was listed on Euronext Amsterdam from June 2007 through April 2012. This period provided company-specific historical and implied volatility information. Since the de-listing of AMT in April 2012, we have not had the same level of company-specific historical and implied volatility information; therefore, we estimate the expected volatility based on the

historical volatility of publicly traded peer companies with a similar focus on gene therapies, biological products or orphan diseases, including Oxford Biomedica plc, MolMed S.p.A., Transgene SA, Sarepta Therapeutics, Inc., Sangamo Biosciences Inc. and Synageva BioPharma Corp.

We account for share options as an expense in the statement of comprehensive income over the estimated vesting period, with a corresponding contribution to equity. See Note 10 to our audited consolidated financial statements included elsewhere in this prospectus for a discussion of the total expense recognized in the statement of comprehensive income for share options granted to employees, supervisory board members and consultants.

The following table summarizes, by grant date, the number of ordinary shares underlying share options granted from January 1, 2012 through September 30, 2013, as well as the associated per share exercise price, the estimated fair value per ordinary share on the grant date, the retrospective estimated fair value per share on the grant date, and the estimated fair value per option as of the grant date:

GRANT DATE	NUMBER OF ORDINARY SHARES UNDERLYING OPTIONS GRANTED	EXERCISE PRICE PER ORDINARY SHARE	ESTIMATED FAIR VALUE PER ORDINARY SHARE AT GRANT DATE	RETROSPECTIVE FAIR VALUE PER ORDINARY SHARE AS OF GRANT DATE <sup>(1)</sup>	ESTIMATED FAIR VALUE PER OPTION AS OF GRANT DATE
April 5, 2012	6,831,561	€ 0.614	€ 0.614	€ 0.614	€ 0.41
June 12, 2012	75,000	0.614	0.614	0.614	0.41
December 1, 2012	703,260	0.614	0.614	0.97	0.67
December 22, 2012	421,956	0.614	0.614	1.02	0.72
January 1, 2013	560,000	1.00	1.00	1.09	0.68
March 26, 2013	70,326	1.00	1.00	1.53	1.06
June 5/6, 2013	140,000	2.02	2.02	2.52	1.63
September 1, 2013	703,260	2.02	2.66	N/A	1.77

<sup>(1)</sup> The fair value of our ordinary shares at the grant date was adjusted in connection with our retrospective fair value assessment for financial reporting purposes, as described below.

Of the 8,451,110 options which have been granted and remained outstanding at September 30, 2013, an aggregate of 2,391,085 options were granted to members of the management board. 7,352,225 options which have been granted and remained outstanding at September 30, 2013 will vest in full upon the closing of this offering, which would result in the acceleration of any unrecognized expense related to these options. As of September 30, 2013, the unrecognized expense related to the options which have been granted and remained outstanding at September 30, 2013 was €2.2 million.

The intrinsic value of all outstanding vested and unvested options as of September 30, 2013 was \$ , based on an assumed public offering price of \$ per share, which is the midpoint of the price range set forth on the cover page of this prospectus, and was based on ordinary shares issuable upon the exercise of options outstanding as of with a weighted average exercise price of € per share.

#### ***Determination of the Fair Value of Ordinary Shares on Grant Dates***

We are a private company with no active public market for our ordinary shares. Therefore, we have periodically determined for financial reporting purposes the estimated per share fair value of our ordinary shares at various dates using contemporaneous valuations. We performed these contemporaneous valuations as of each of the grant dates identified above. In conducting the contemporaneous valuations, our management board and supervisory board considered all objective and subjective factors that we believed to be relevant for each valuation conducted, including their best estimate of our business condition, prospects and operating performance at each valuation date.

In connection with the preparation of our audited financial statements for 2012, our management board and supervisory board reconsidered the fair values as of each grant date through September 2013, and engaged an independent third party to conduct a retrospective fair value assessment as of each grant date, as described below, for financial reporting purposes. In light of management's retrospective assessment of the various grants, the estimated fair values and, accordingly, the related compensation expense, were adjusted as appropriate.

There are significant judgments and estimates inherent in the determination of fair value of our ordinary shares. These judgments and estimates include assumptions regarding our future operating performance, the time to completing an initial public offering or other liquidity event and the determinations of the appropriate valuation methods. If we had made different assumptions, our share-based compensation expense, net loss and net loss per ordinary share could have been significantly different.

Our management board and supervisory board consider numerous objective and subjective factors to determine their best estimate of the fair value of the ordinary shares as of each grant date, including the following:

- the progress of our research and development programs;
- achievement of enterprise milestones, including the entering into of collaboration and license agreements;
- contemporaneous issuances and valuations of our ordinary shares;
- our historical and forecasted performance and operating results;
- our need for future financing to fund operations;
- the likelihood of our achieving a discrete liquidity event, such as a sale of our company or an initial public offering, given prevailing market conditions;
- the dilutive effect of employee incentive instruments, our convertible loan and warrants; and
- external market and economic conditions impacting our industry.

In determining the estimated fair values of our ordinary shares as of each award grant date, three generally accepted approaches were considered: income approach, market approach and cost approach. Based on our stage of development and information available, we have determined that the income approach is the most appropriate method. When applicable, we have also applied the market approach by employing recent sales of company shares as a method to estimate our aggregate enterprise value. In addition, we have taken into consideration the guidance prescribed by the American Institute of Certified Public Accounts, or AICPA, Audit and Accounting Practice Aid, Valuation of Privately-Held-Company Equity Securities Issued as Compensation.

Discounted cash flow, or DCF, an income approach to determining the fair value of the ordinary shares, which we estimated as of each award grant date, is an estimate of the present value of the future monetary benefits expected to flow to the owners of a business. It requires a projection of the cash flows that the business is expected to generate. These cash flows are converted to present value by means of discounting, using a rate of return that accounts for the time value of money and the appropriate degree of risks inherent in the business. The discount rate in the DCF analysis is based upon a weighted average cost of capital, or WACC, calculated at each valuation date. The WACC is a method that market participants commonly use to price securities and is derived by using the capital asset pricing model and inputs such as the risk-free rate, beta coefficient, which is a measure of the sensitivity of a share price to movements in the overall securities market, equity risk premiums and the size of the company. We analyzed our financial and operating projections, including revenues, operating expenses, working capital investments and capital expenditures, to form the basis for our DCF valuation.

In applying the Black-Scholes option model, we applied a discount for lack of marketability, or DLOM, to reflect the increased risk arising from the inability to readily sell the ordinary shares underlying the options

granted. Under this method, we considered the cost of the put option, which can hedge the price change before the privately held ordinary shares can be sold, as the basis to determine the DLOM. The cost of the put option was the only factor we considered and applied in the discount. The put option analysis reflects the potential loss from marketability over the expected time to liquidity and is a commonly applied approach to estimate this discount.

We have also considered prior arm's length sales of our equity securities in order to estimate our business enterprise value. Considerations factored into the analysis include the type and amount of equity sold, the relationship of the parties involved, the timing compared to the ordinary share valuation date and the financial condition and structure of a company at the time of the sale.

In the retrospective valuation described above, three key valuation inflection points were identified during the period from April 2012 through September 2013:

- On July 20, 2012, the CHMP issued a positive recommendation for the approval of our marketing authorization application for Glybera.
- On October 31, 2012, the European Commission formally approved Glybera for a subset of LPLD patients.
- On April 29, 2013, we signed an agreement with Chiesi for the commercialization of Glybera in the European Union and other specified countries.

We believe that each of these events had a significant positive effect on the fair value of our ordinary shares, and served as references points for the determination of fair value at each grant date. In particular, developments between October 2012 and April 2013 reduced both execution and financing risks facing our business. For grants made between these value inflection points and between April and September 2013, we interpolated the key valuation assumptions, including the probability of success of our individual development programs and the weighted average cost of capital as of such dates.

#### *Share Option Grants on April 5, 2012*

AMT shares were previously listed on Euronext Amsterdam. On the five business days immediately prior to February 17, 2012, the date on which we announced our proposed acquisition of the entire business and assets of AMT, the average closing price of the shares of AMT on Euronext Amsterdam was €0.614 per AMT share. Given that uniQure had no other business of its own, and that the consideration for purchase of the business and assets of AMT was a one-for-one share issuance in respect of each AMT share then in issue, we believed this represented the fair value of our ordinary shares as of this date. In addition, on April 5, 2012, we raised €6.0 million through the issuance of new shares to an existing shareholder at a price per ordinary share of €0.614.

All AMT options outstanding at the time we acquired the assets and business of AMT were terminated. In order to retain and provide incentives for our employees following this corporate reorganization, we granted options to purchase an aggregate of 6,831,561 ordinary shares on April 5, 2012, with an exercise price of €0.614 per share. Our management board and supervisory board determined that €0.614 represented the fair value per ordinary shares as of the grant date based on the average closing price of AMT shares on Euronext Amsterdam on the five days immediately prior to the announcement of the acquisition of the business and assets of AMT by uniQure and the concurrent equity investment transaction.

#### *Share Option Grants on June 12, 2012*

On April 19, 2012, the EMA announced that, following a request from the European Commission in January 2012, it had re-evaluated Glybera in a restricted group of patients with severe or multiple pancreatitis attacks. The EMA maintained its previous recommendation that Glybera should not be granted marketing authorization at that time.



On April 18, 2012, we raised €1.0 million through the issuance of new shares to an existing shareholder at a price of €0.614 per ordinary share. Our management board and supervisory board determined that, although we had raised sufficient new capital in our financing transaction in April 2012 to continue as a going concern, we did not have enough capital at that time to progress the development and commercialization of Glybera. Therefore, the issuance price for the April 2012 equity financing was unchanged from the issuance price used in the April equity financing. Accordingly, our management board and supervisory board determined that €0.614 remained the estimated fair value of our ordinary shares at that date based on this third party equity financing transaction.

On June 12, 2012, we granted options to purchase an aggregate of 75,000 ordinary shares at an exercise price of €0.614 per share. Our management board and supervisory board determined that there had been no meaningful change in our financial position or performance between April 18, 2012 and this grant date to warrant a change in the estimated fair value of our ordinary shares.

#### *Share Option Grants on December 1, 2012*

On July 20, 2012, the CHMP recommended approval of Glybera for the restricted population of LPLD patients with severe or multiple pancreatitis attacks, subject to additional post-marketing studies for efficacy. We view this as the first of three key value inflection points at which we observed a significant change in our company and per share value. Although there was still significant uncertainty about Glybera's formal approval in the European Union and our ability to proceed, we believe that this marked an increase in our company value.

On October 31, 2012, the second value inflection point, the European Commission formally approved the marketing authorization for Glybera under exceptional circumstances. This removed further uncertainty around formal approval of Glybera.

Notwithstanding the positive regulatory developments in the European Union for Glybera, during 2012 and the first nine months of 2013, our management board and supervisory board considered that any increase in the value of our company following the approval of Glybera was partially offset by the substantial increase in financial risk, including uncertainty as to whether we would be able to continue as a going concern. Accordingly, to calculate our company valuation at each value inflection point, we performed an analysis, revising the value drivers to account for the changes in probability of success of our pipeline product candidates in light of the European Union regulatory developments surrounding Glybera, changes in the WACC to reflect changes in financing risk and in the probability of success, our cash burn, and the discounting effect of cash flows.

We used these outcomes to estimate the fair value of option grants on December 1, 2012; December 22, 2012; January 1, 2013; March 26, 2013 and June 5/6, 2013 using the Black-Scholes option pricing model. Use of this valuation methodology requires that we make assumptions as to the value of the underlying asset, the exercise price, the expected volatility of share price returns, which is estimated based on the observed average of the daily share price returns of selected guideline companies measured over a historic period equal to the expected term of our share options and the risk free interest rate for a period that approximates the expected term of our share options and our expected dividend yield.

On December 1, 2012, we granted options to purchase an aggregate of 703,260 ordinary shares with an exercise price of €0.614 per share, which our management board and supervisory board contemporaneously determined to be the fair value of our ordinary shares as of such date.

Our management board and supervisory board subsequently commissioned a retrospective valuation of these ordinary shares, considering our financial risk, and arrived at an estimated fair value per share of €0.97. We relied on the DCF method to perform the retrospective valuation as of December 1, 2012. Our key assumptions included probabilities of success of 65 to 85% and a WACC of 21%. We applied a DLOM of 20%. Based on estimated value per ordinary share of €0.97, this resulted in the fair value per option of €

0.66 to €0.68. The key assumptions we used to arrive at the estimated value per option included an estimated volatility of 70%, an expected life of 5.5 to 6.3 years, and a risk-free rate of 0.6% to 0.7% based on the yield to maturity of German sovereign debt, for a term equal to the expected life as measured on the grant date. This increase from the prior valuation performed in June 2012 primarily related to the European Commission's formal approval of the marketing authorization for Glybera and the resulting increased probability of success of our Glybera program. For financial reporting purposes, this value has been applied retrospectively to our December 1, 2012 option grants.

*Share Option Grants on December 22, 2012*

On December 22, 2012, we granted options to purchase an aggregate of 421,956 ordinary shares with an exercise price of €0.614 per share, which our management board and supervisory board contemporaneously determined to be the fair value of our ordinary shares as of such date.

Our management board and supervisory board subsequently commissioned a retrospective valuation of these ordinary shares, considering our financial risk, and arrived at an estimated fair value per share of €1.02. We relied on the DCF method to perform the retrospective valuation as of December 22, 2012. Key assumptions were unchanged relative to the December 1, 2012 valuation date, including probabilities of success of 65 to 85% and a WACC of 21%. We applied a DLOM of 15%. Based on estimated value per ordinary share of €1.02, this resulted in the fair value per option of €0.71 to €0.73. The key assumptions we used to arrive at this estimated fair value per option included an estimated volatility of 70%, an expected life of 5.5-6.3 years, and a risk-free rate of 0.5% to 0.7% based on the yield to maturity of German sovereign debt, for a term equal to the expected life as measured on the grant date. This increase from the prior valuation primarily reflected the increased probability of success of our Glybera program and additional funding received during December 2012. For financial reporting purposes, this value has been applied retrospectively to our December 22, 2012 option grants.

*Share Option Grants on January 1, 2013*

On January 1, 2013, we granted options to purchase an aggregate of 560,000 ordinary shares with an exercise price of €1.00 per share, which our management board and supervisory board contemporaneously determined to be the fair value of our ordinary shares as of such date.

Our management board and supervisory board subsequently commissioned a retrospective valuation of these ordinary shares, considering our financial risk, and arrived at an estimated fair value per share of €1.09. We relied on the DCF method to perform the retrospective valuation as of January 1, 2013. Key assumptions were unchanged relative to the December 22, 2012 valuation date, including probabilities of success of 65 to 85% and a WACC of 21%. We applied a DLOM of 15%. Based on estimated value per ordinary share of €1.09, this resulted in the fair value per option of €0.67 to €0.70. The key assumptions we used to arrive at the estimated fair value per option included an estimated volatility of 70%, an expected life of 5.5 to 6.3 years, and a risk-free rate of 0.4% to 0.6% based on the yield to maturity of German sovereign debt, for a term equal to the expected life as measured on the grant date. This increase from the previous valuation primarily reflected the progress we had made by that date in identifying a potential collaborator for the commercialization of Glybera. For financial reporting purposes, this value has been applied retrospectively to our January 1, 2013 option grants.

*Share Option Grants on March 26, 2013*

On March 26, 2013, we granted options to purchase an aggregate of 70,326 ordinary shares with an exercise price of €1.00 per share, which our management board and supervisory board contemporaneously determined to be the fair value of our ordinary shares as of such date.

Our management board and supervisory board subsequently commissioned a retrospective valuation of these ordinary shares, considering our financial risk, and arrived at an estimated fair value per share of €1.53. We

relied on the DCF method to perform the retrospective valuation as of March 26, 2013. Key assumptions included increased probabilities of success of 70 to 90% due to technical progress achieved and a WACC of 18.9% due to decreased financing risk since the January 1, 2013 valuation date. We applied a DLOM of 15%. Based on estimated value per ordinary share of €1.53, this resulted in the fair value per option of €1.04 to €1.08. The key assumptions we used to arrive at the estimated fair value per option included an estimated volatility of 70%, an expected life of 5.5 to 6.3 years, and a risk-free rate of 0.5% to 0.6% based on the yield to maturity of German sovereign debt, for a term equal to the expected life as measured on the grant date. This increase in the fair value of our ordinary shares primarily reflected additional financing received since the prior valuation, which provided us with the funds necessary to continue the development of Glybera. For financial reporting purposes, this value has been applied retrospectively to our March 26, 2013 option grants.

#### *Share Option Grants on June 5/6, 2013*

On April 29, 2013, the third value inflection point, we entered into an agreement with Chiesi with respect to the commercialization of Glybera in the European Union and selected other countries. This agreement was subject to the satisfaction by us of specified conditions precedent. The Chiesi transaction allowed us to update the estimate of the present value of our expected future cash flows based on the value of the transaction.

On June 5, 2013 and June 6, 2013, we granted options to purchase an aggregate of 140,000 ordinary shares with an exercise price of €2.02 per share, which our management board and supervisory board contemporaneously determined to be the fair value of our ordinary shares as of such date.

Our management board and supervisory board subsequently commissioned a retrospective valuation of these ordinary shares, considering our financial risk, and arrived at an estimated fair value per share of €2.52. We primarily relied on the expected transaction price of the ordinary shares subsequently acquired by Chiesi pursuant to the conditional agreements entered into in April 2013, of €2.52 per share to perform the retrospective valuation as of June 5/6, 2013. Additionally, we performed a DCF as of June 2013. Key assumptions included probabilities of success of 70 to 90% and a WACC of 16.0%. We applied a DLOM of 15%. The probabilities of success remained unchanged relative the prior valuation date, while the WACC was lowered to reflect a further decrease in financing risk given the additional funding provided by Chiesi. Based on estimated value per ordinary share of €2.52, this resulted in the fair value per option of €1.60 to €1.67. The key assumptions we used to arrive at the estimated fair value per option included an estimated volatility of 70%, an expected life of 5.5 to 6.3 years, and a risk-free rate of 0.7% to 0.8% based on the yield to maturity of German sovereign debt, for a term equal to the expected life as measured on the grant date. This increase in the fair value of the ordinary shares was primarily due to the announcement that we would be entering into collaboration agreements with Chiesi, including the issuance to Chiesi of 8.75% of our share capital. For financial reporting purposes, this value has been applied retrospectively to our June 5/6, 2013 option grants.

#### *Share Option Grants on September 1, 2013*

On September 1, 2013, we granted options to purchase an aggregate of 703,260 ordinary shares with an exercise price of €2.02 per share.

Our management board and supervisory board contemporaneously commissioned a valuation of the ordinary shares, and arrived at an estimated fair value per share of €2.66 as of the grant date. We performed a DCF as of September 1, 2013. Key assumptions included probabilities of success of approximately 70 to 90% and a WACC of 16.0%. We applied a DLOM of 10%. The probabilities of success remained unchanged relative to the prior valuation date. Based on estimated value per ordinary share of €2.66, this resulted in the fair value per option of €1.74 to €1.81. The key assumptions we used to arrive at the estimated fair value per option included an estimated volatility of 70%, an expected life of 5.5 to 6.3 years, and a

risk-free rate of 1.0% to 1.2% based on the yield to maturity of German sovereign debt, for a term equal to the expected life as measured on the grant date. This increase in the fair value of the ordinary shares was primarily due to passage of time, such that positive operating cash flows come nearer.

## **Recent Accounting Pronouncements**

There are no IFRS standards as issued by the IASB or interpretations issued by the IFRS interpretations committee (e.g. IFRS 10, 11, 12, 13 and IAS 19R) that are effective for the first time for the financial year beginning on or after January 1, 2013 that had or are expected to have a material impact on our financial position.

## **JOBS Act Exemptions**

On April 5, 2012, the Jumpstart Our Business Startups, or JOBS, Act was signed into law. The JOBS Act contains provisions that, among other things, reduce certain reporting requirements for an "emerging growth company." As an emerging growth company, we are electing to take advantage of the following exemptions:

- including the use of two years of audited financial statements rather than three years;
- not providing an auditor attestation report on our system of internal controls over financial reporting;
- not providing all of the compensation disclosure that may be required of non-emerging growth public companies under the U.S. Dodd-Frank Wall Street Reform and Consumer Protection Act; and
- not complying with any requirement that may be adopted by the Public Company Accounting Oversight Board regarding mandatory audit firm rotation or a supplement to the auditor's report providing additional information about the audit and the financial statements (auditor discussion and analysis).

The JOBS Act permits an "emerging growth company" such as us to take advantage of an extended transition period to comply with new or revised accounting standards applicable to public companies. We are irrevocably electing not to avail ourselves of this extended transition period for complying with new or revised accounting standards and, therefore, we will be subject to the same new or revised accounting standards as other public companies that are not "emerging growth companies."

These exemptions will apply for a period of five years following the completion of our IPO or until we no longer meet the requirements of being an "emerging growth company," whichever is earlier. We would cease to be an emerging growth company if we have more than \$1.0 billion in annual revenue, have more than \$700 million in market value of our ordinary shares held by non-affiliates or issue more than \$1.0 billion of non-convertible debt over a three-year period.

## BUSINESS

### Overview

We are a leader in the field of gene therapy and have developed the first and currently the only gene therapy product to receive regulatory approval in the European Union. Gene therapy offers the prospect of long-term and potentially curative benefit to patients with genetic or acquired diseases by directing the expression of a therapeutic protein or restoring the expression of a missing protein through a single administration. Our first product, Glybera, was approved by the European Commission in October 2012 under exceptional circumstances for the treatment of a subset of patients with LPLD, a potentially life-threatening, orphan metabolic disease. We expect to launch Glybera commercially in selected European countries in the first half of 2014 through our collaboration with Chiesi, which we entered into in April 2013. We retain full commercial rights to Glybera in the United States. In August and December 2013, we met with the FDA to discuss the regulatory pathway for Glybera in the United States and we plan to file an IND with the FDA for Glybera in the first half of 2014.

We are developing a pipeline of additional AAV-based gene therapies through multiple collaborations that are designed to accelerate the development and commercialization of these programs. We develop our gene therapies using our innovative, modular technology platform, including our proprietary, cost-effective manufacturing process. Our pipeline includes product candidates targeting diseases for which either the efficacy of existing treatments is limited or the administration regimen is burdensome, such as hemophilia B, as well as diseases for which there are currently no treatments, such as Sanfilippo B syndrome. We initially intend to focus on orphan diseases but believe we will also be able to leverage our technology to develop gene therapies targeting chronic and degenerative diseases that affect larger populations. Through our gene delivery system know-how, our proprietary manufacturing process, the state-of-the-art facility we are building out and equipping in the United States, and our experience in developing and obtaining regulatory approval for Glybera in the European Union, we believe we will be able to develop and commercialize additional gene therapies more efficiently than our competitors.

Our gene therapy approach seeks to treat the causes of genetic diseases by enabling patients to effectively express a missing or deficient protein. To accomplish this, Glybera and our product candidates are designed to deliver a transgene through a delivery system called a vector. Our approach is designed to be modular, in that it may allow us to develop, manufacture and seek regulatory approval for multiple gene therapies generally using the same principal components. The key components of our gene therapy approach are:

- the therapeutic gene cassettes we design or in-license from academic research institutions and biotechnology and pharmaceutical companies, including our collaborators;
- an AAV-based vector delivery system with a demonstrated safety profile that selectively targets relevant tissues;
- administration technologies designed to optimize the introduction of our gene therapy vectors into the patient's body; and
- our scalable, proprietary manufacturing process.

Glybera is indicated for the treatment of adult patients diagnosed with familial LPLD confirmed by genetic testing and suffering from severe or multiple pancreatitis attacks despite dietary fat restrictions. LPLD results in hyper-chylomicronemia, or dramatic and potentially life-threatening increases in the level of large fat-carrying particles, called chylomicrons, in the blood after eating. In many cases, LPLD and the associated elevated levels of chylomicrons can cause acute and potentially life-threatening inflammation of the pancreas, known as pancreatitis, thus leading to frequent hospitalizations. Recurrent pancreatitis can lead to chronic abdominal pain, pancreatic insufficiency, which is an inability to properly digest food due to a lack of digestive enzymes made by the pancreas, and diabetes.

Prior to Glybera, there has been no approved therapy for the treatment of LPLD. Patients with this rare condition are required to adhere to a strict low-fat diet and to abstain from alcohol. These restrictions, as well as frequent hospitalizations and the constant fear of encountering pancreatitis attacks, have a significant negative impact on the daily activity level of LPLD patients and on their quality of life.

In addition to Glybera, our development pipeline includes our internal program for hemophilia B, two collaborator-directed programs for monogenic diseases, one collaborator-directed program for a degenerative disease and several programs in early preclinical development. The most advanced of these pipeline programs are the following:

**Internal program: AMT-060 for Hemophilia B.** In collaboration with Chiesi, we are developing AMT-060 for the treatment of hemophilia B, which is a severe orphan blood clotting disorder. AMT-060 consists of an AAV5 vector carrying a therapeutic hFIX gene cassette that we have exclusively licensed from St. Jude. We plan to file an IND and an IMPD to initiate a Phase I/II dose escalation clinical trial of this product candidate in the second half of 2014 in 13 to 16 patients. St. Jude is currently conducting a Phase I/II clinical trial in this indication with a gene therapy consisting of an AAV8 vector carrying the same hFIX gene that we are using in AMT-060. We describe these as both Phase I and Phase II clinical trials because their goals are not merely to evaluate the therapy's safety but also to obtain a preliminary determination of efficacy.

**Collaborator-sponsored programs.** We are also collaborating with third parties that are sponsoring early-stage clinical trials of gene therapy product candidates to which we hold certain rights. We believe that this approach enables us to cost-effectively obtain access to preclinical and early-stage clinical results without expending significant resources of our own. These programs utilize either clinical materials that we have manufactured as part of our collaborations or gene cassettes that we have licensed. We generally have the rights to the data generated in these collaborator-sponsored clinical development programs, but do not control their design or timing. If we decide to progress any of these programs internally, we may need to develop or in-license additional technology. The most advanced of these programs are the following:

- **AMT-021 for Acute Intermittent Porphyria.** We and our collaborator Digna Biotech are developing AMT-021 as a gene therapy for AIP, a severe liver disorder. AMT-021 consists of an AAV5 vector carrying a therapeutic PBGD gene that we exclusively license from Applied Medical Research Center of the University of Navarra in Spain, or CIMA. Digna Biotech is currently conducting a Phase I clinical trial of AMT-021 in eight patients in Spain. We have manufactured the gene therapy being used in this clinical trial. We understand from Digna Biotech that data are expected in the second half of 2014. Under an agreement with Digna Biotech, we have an exclusive right to use all preclinical and Phase I clinical trial data from this program.
- **AMT-110 for Sanfilippo B Syndrome.** We and our collaborator Institut Pasteur are developing AMT-110 as a gene therapy for Sanfilippo B syndrome, a potentially fatal lysosomal storage disease that results in serious brain degeneration in children. This gene therapy consists of an AAV5 vector carrying a therapeutic NaGLU gene. Institut Pasteur is currently conducting a Phase I/II clinical trial of AMT-110 in four patients in France. We have manufactured the gene therapy being used in this clinical trial. We understand from Institut Pasteur that data are expected in the first half of 2015.
- **AAV2/GDNF for Parkinson's Disease.** We and our collaborator UCSF are developing a gene therapy for Parkinson's disease, a progressive neurodegenerative disorder affecting motor skills, speech and other neurological functions and resulting in progressive neurologic dysfunction and death. UCSF is collaborating with the NIH to conduct a Phase I clinical trial of a gene therapy in this indication that consists of an AAV2 vector manufactured by a third party using a mammalian cell-based process, carrying a therapeutic gene we have exclusively licensed in the gene therapy field from Amgen that expresses GDNF. The NIH is funding and sponsoring the clinical trial. We have rights to the clinical data from this trial. Based on the results of the UCSF study, we may decide to develop an

AAV2-based gene therapy containing the GDNF gene manufactured with our insect cell-based manufacturing process.

We operate a cGMP-manufacturing facility at our headquarters in Amsterdam, the Netherlands, which the EMA has approved for clinical and commercial-grade production. We have also begun the build-out of a 53,000 square-foot manufacturing facility in Lexington, Massachusetts, in the United States, which we believe will be the world's most advanced dedicated facility for the production of AAV-based gene therapies.

## Gene Therapy Background

Genes are the specific areas of DNA that provide the blueprint used by the body's cellular machinery to make proteins, which are enzymes or other large molecules in the cells of the body that serve a functional purpose. Each gene consists of a specific sequence that encodes for the production of specific proteins. This protein production process in the cell is called gene expression.

A mutation, or defect, in a specific gene can result in the inability or reduced ability to express a protein, or the reduced functionality of a protein. For example, when the gene associated with the production of a protein required for blood clotting is missing or mutated in hemophilia B patients, sufficient blood clotting will not occur, resulting in continued internal or external bleeding after even minor trauma or surgery. Introducing a copy of the proper gene into the cell would restore the presence and natural function of the blood clotting factor, which would prevent bleeding.

A large number of serious diseases result from a defect in a single gene. For many of these monogenic diseases, there is currently no cure or therapy. In other cases, existing therapies involve burdensome administration or frequent readministration, and may provide limited efficacy. For example, in the case of hemophilia B, most patients must receive frequent intravenous administration of plasma-derived or recombinant human Factor IX, currently two to three times per week, which often does not completely prevent bleeding. Millions of patients rely on recurrent medical care to help manage their life-long chronic ailments, often at significant cost and, depending upon the disease, with little chance of sustained success.

More than 30 years ago, scientists began exploring the concept of gene therapy as an approach to treating disease. The goal of gene therapy is to treat the underlying genetic cause of disease by introducing a functional gene to replace or supplement the activity of a missing or mutated gene. Gene therapy approaches include *in vivo* therapies, in which the functional gene is introduced directly into the patient's body, and *ex vivo* therapies, in which a patient's cells are removed, genetically modified and then transplanted back into the patient.

To enable delivery of the functional gene into the cells where it can produce the required protein, researchers use a vector that can enter, or transduce, the cell without harming it. Researchers generally consider vector systems derived from viruses to be more efficient in entering the target cells than non-viral vector systems. In addition, to target the vector to the tissues for the relevant disease, researchers using AAV-based vectors utilize different variants, or serotypes, of AAV to selectively target particular tissues. The vectors are designed to include DNA promoters, which are additional DNA sequences that allow for tissue-specific expression of the required protein.

We believe that most research and development efforts in gene therapy have focused on monogenic diseases, which include many hereditary diseases. Although these diseases are severe, their cause is generally better understood and less complex than diseases that involve more than one dysfunctional or missing gene. There are also opportunities to apply gene therapy in diseases caused by more complex pathology in which one particular protein plays a crucial role in the causation of the disease. In such indications, such as some liver diseases, disorders of the central nervous system and cardiovascular diseases, it may be possible to halt or eradicate the disease with a gene therapy that promotes the natural production or function of the relevant protein. Gene therapy also has the potential to neutralize faulty genes, referred to as gene silencing, and to switch genes on or off.



We believe that as scientific understanding of the genetic causes of disease expands and as genetic sequencing becomes more cost-efficient and routine, the practice of medicine will increasingly turn to gene therapy as an approach to treating, and potentially preventing, disease, with resulting longer-term cost benefits to the health care system.

## Historical Challenges Faced By Gene Therapy

Gene therapy has historically confronted a number of significant challenges, including the following:

- **Poor Safety Profile.** Early gene therapies raised significant safety concerns. For example, some *ex vivo* gene therapy approaches use lentiviral or retroviral vectors that can integrate at relatively high frequency into parts of the genome in a manner that increases the risk of cancer. In addition, an early *in vivo* gene therapy approach using an adenovirus vector triggered a strong innate immune response in a patient that resulted in multiple-organ failure and death. These high-profile safety issues resulted in negative public perceptions of the safety of gene therapy generally.
- **Limited Efficacy.** Early gene therapy manufacturing processes produced a large proportion of empty vectors, or viral packages that did not include the therapeutic gene of interest. In addition, because patients' immune systems respond to proteins in the vector shell, the large proportion of empty vectors generated counterproductive immune responses. Limited long-term expression of the delivered genes in target cells also made it difficult to achieve meaningful clinical efficacy in either the short- or long-term. These problems reflected the fact that early researchers had not identified the AAV serotypes, optimized genes or DNA promoters that could target the appropriate tissues and produce levels of gene expression necessary to demonstrate clinical efficacy. Earlier approaches utilizing AAV vectors also typically involved the packaging of single-stranded DNA into delivery vectors. Because the required starting point for the transcription of a gene is double-stranded DNA, this single-stranded approach required the cellular machinery to make a second copy of the DNA, which added an additional step in the process and reduced expression levels.
- **Difficulties in Administration and Tissue-Specific Targeting.** The efficacy of some earlier gene therapies was limited by difficulties in administering the vector to the patient's body in a manner that resulted in effective delivery of the therapeutic transgene into the desired target tissue or organ. For example, researchers have historically been challenged in administering gene therapies targeting CNS indications due to the difficulties presented by the blood-brain barrier. Only recently have scientists begun to optimize the gene therapy administration process through the use of advanced technologies for local administration to muscle cells and neurons.
- **Lack of Cost-Effective and Consistent Manufacturing.** Until recently, AAV-based vectors could not be manufactured at commercial scale on a cost-effective, reliable and reproducible basis. Difficulties in increasing production levels to commercial scale were particularly challenging in manufacturing processes that utilized mammalian cells. These mammalian cell processes typically use adherent cells, which are cells that only grow on a surface. As a result, production of larger quantities of vector required large surfaces, which is more challenging and less economical than manufacturing processes that use cells that can be grown in a suspension culture.

We believe these factors have contributed to the failure to develop approved gene therapy products in the past. Glybera is the only approved gene therapy in the European Union and no gene therapy has been approved to date in North America.

## Our Gene Therapy Approach

### Overview

We believe that our modular technology platform addresses many of the historical challenges of gene therapy. The key components of our gene therapy approach are the following:

- therapeutic gene cassettes;



- AAV-based vector delivery system;
- administration technologies; and
- our scalable, proprietary manufacturing process.

We expect that our modular approach will allow us to use the same building blocks to efficiently develop, manufacture and seek regulatory approval for multiple new gene therapies. In some cases, we believe that the disease-specific gene cassette will be the only component we need to change to target a new disease in a particular tissue. As a result, we may be able to reduce the overall preclinical and potentially clinical development activities required to obtain regulatory approval, and to significantly reduce the overall development risk, time and cost.

### ***Therapeutic Gene Cassettes***

We design our gene therapies to deliver into the body's cells a transgene that encodes, or provides the blueprint for the expression of, a therapeutic protein. The transgene is carried in a gene cassette together with DNA promoters that direct expression in specific tissues. We either develop the gene cassettes we use or in-license them, often as part of our collaborations with academic research institutions and biotechnology and pharmaceutical companies. In-licensing gene cassettes provides us access to key intellectual property and allows us to build upon our collaborators' scientific expertise and financial investment, as well as their preclinical and, in some cases, clinical development efforts.

### ***Our AAV-based Vector Delivery System***

We deliver the gene cassette to the target tissue using an engineered, non-replicating viral vector delivery system based on AAV. We have based Glybera and all of our current product candidates on our AAV-based vector technologies, which we believe address many of the safety and efficacy challenges that hindered earlier gene therapy approaches.

*Demonstrated Safety Profile.* AAV is one of several viruses commonly used as a vector in gene therapy. A significant proportion of people have already been exposed to AAV in the normal course of their lives. AAV-based vectors cause only a mild immune response, including innate responses immediately following treatment. Regulatory agencies in the United States and European Union have extensive experience reviewing AAV-based vectors. In preclinical research and more than 80 gene therapy clinical studies conducted by us or third parties, AAV-based vectors have demonstrated a good safety profile.

Unlike retrovirus and lentivirus vectors, which are other vectors commonly used in gene therapies, AAV vectors integrate into the host genome only at low frequency, which substantially reduces the risk of adverse effects, including cancer. As a further safety measure, we engineer our AAV vectors without any components required for the virus to replicate and infect additional cells. We also believe the purification step in our manufacturing process enables us to optimize the purity of our vector material, which reduces the risk of side effects.

*Improved Gene Expression.* For a gene therapy to be effective, it must provide lasting therapeutic gene expression in the target tissue. AAV-based vectors have shown persistent effects in animal experiments and in clinical studies. AAV-based vectors have also demonstrated sustained expression in the target tissue of animal models for more than ten years. St. Jude has reported expression in target tissue in humans for more than three years after a single treatment in its ongoing Phase I/II clinical trial of a gene therapy for the treatment of hemophilia B.

We use different serotypes of AAV to selectively target particular tissues. We use AAV1 in Glybera, for example, because we believe it is particularly suited to targeting myocytes, a type of cell found in muscle tissue. We have based most of our pipeline projects on AAV5, which we believe has a strong tropism for both hepatocytes, a type of cell found in the liver, and neurons, a type of cell found in the central nervous system. We hold an exclusive license to three patents owned by the NIH for the development and sale of

AAV5-based therapeutic products to be delivered to the brain or liver for the treatment of human diseases originating in the brain or liver, excluding arthritis related diseases, and a non-exclusive license to those patents for the development and sale of AAV5-based therapeutic products to treat other human diseases. In addition, we are using AAV2 as a vector for gene therapies that are delivered to the brain.

### **Administration Technologies**

We and our collaborators are developing expertise in utilizing a variety of technologies to administer the vector to the body in order to optimize delivery of our gene therapies into the tissues or organs relevant to the indication we are targeting. These include intramuscular injection of AAV1 vectors that deliver a transgene into muscle cells for Glybera, intravenous infusion of AAV5 vectors that deliver a transgene to liver cells for our hemophilia B program, and intracranial administration of AAV5 and AAV2 vectors for delivery of a transgene to cells within the brain for Sanfilippo and Parkinson's disease, respectively. In the case of diseases of the CNS, in particular, we believe that the effectiveness of gene therapy will depend upon both the choice of vector and the mode of administration of the vector. For example, our collaborator UCSF is currently conducting a Phase I clinical trial of a gene therapy for Parkinson's disease using MRI guided convection-enhanced delivery to the brain. Convection-enhanced delivery involves placement of one or more catheters directly into the brain to provide distribution of the therapeutic agent to a confined region, such as the putamen, in order to provide adequate drug concentrations directly to the relevant tissue. We believe that this may represent a significant improvement over administration methods used in prior clinical trials of gene therapies targeting the brain. In other CNS indications, widespread delivery of a transgene is preferable. In preclinical animal models, we have shown widespread distribution of AAV5 in the brain when administered via the cerebral spinal fluid.

### **Scalable, Proprietary Manufacturing**

We produce our AAV-based gene therapies in our own facility with our proprietary manufacturing process, which uses insect cells and baculoviruses, a common family of viruses found in invertebrates. Our insect-cell based manufacturing process, which uses cells that can be grown in a suspension culture, is designed to produce higher yields of vectors more cost-effectively and efficiently than the mammalian cell-based approaches that many of our competitors utilize. We non-exclusively license from the NIH the use of baculoviruses and insect cells in the production of AAV-based vectors, and have augmented this licensed technology with patented improvements to the replication process designed to allow us to produce gene therapies at commercial scale. The key steps in our proprietary manufacturing process are the following:

- We use a gene cassette containing the therapeutic transgene together with the appropriate promoter and other DNA components required for replication and packaging by the AAV vector.
- We insert the gene cassette into the genome of a baculovirus, which we use as an engineering tool in our manufacturing process to generate the AAV particles that are ultimately used for the therapeutic intervention.
- We then infect insect cells derived from *Spodoptera frugiperda*, the caterpillar stage of the fall army worm moth, with the baculovirus containing the gene cassette.
- At the same time, we infect these cells with two additional baculoviruses containing the elements needed for the proteins of the shell, or capsid, of the AAV vector, and the replication proteins required to create multiple copies of the gene cassette that are subsequently packaged into the AAV capsids. The replication proteins we use incorporate our patented modifications to increase the efficiency of production, and in particular to effect an increase in the proportion of particles containing the therapeutic gene cassette, rather than empty vectors, and to reduce impurities. Our patented modifications also make it possible to efficiently package the equivalent of double-stranded, or self-complementary, DNA into the capsids, which results in increased protein expression levels from the transgene compared with single-stranded DNA.

- We incubate the cells infected with the three different baculoviruses for three days. During this time, the cells produce large numbers of the AAV vector particles containing the gene cassette.
- After three days, we harvest the cells and treat them with a solution known as a lysis buffer to burst the insect cells, which releases the AAV vectors.
- We then purify, concentrate and filter the AAV vectors to yield a pure, high-grade AAV vector suitable for use in therapeutic interventions.

We have begun commercial-scale production of Glybera and expect that once the build-out of our Lexington, Massachusetts facility is complete, our manufacturing process and facilities will enable us to produce Glybera and many other gene therapies cost-effectively at commercial scale.

## Our Strategy

Our strategic goal is to transform the paradigm of care for many severe and chronic diseases by moving from the short-term management of symptoms to potentially curative resolution of the disease through sustained therapeutic gene expression in target tissues. We are building on the capabilities that have enabled us to obtain the first regulatory approval of a gene therapy in the European Union to address a range of diseases for which we believe we can reach the market with a gene therapy ahead of our competitors. We seek to achieve our goal by pursuing the following key objectives:

- **Maximize the value of Glybera.** We are working with our collaborator Chiesi to commercially launch Glybera in the European Union in the first half of 2014. We and Chiesi also plan to seek additional marketing approvals for Glybera in countries outside the European Union that are covered by this collaboration. We have agreed to manufacture and supply to Chiesi its commercial requirements of Glybera. We are working with Chiesi to establish a gene therapy pricing and business model for Glybera that is designed to capture the significant value we believe Glybera delivers to patients and the healthcare system. We believe that our collaboration with Chiesi will enable us to efficiently access markets in which Chiesi has a commercial presence. We are also applying our experience in obtaining EMA approval of Glybera to our development strategy in the United States. We met with the FDA in August and December 2013 to discuss the regulatory pathway for Glybera and we plan to file an IND with the FDA for Glybera in the first half of 2014. If we receive regulatory approval from the FDA, we currently plan to market Glybera in the United States ourselves. Outside the United States and the Chiesi territory, we intend to determine on a case by case basis whether to pursue regulatory approval and commercialize Glybera on our own or to do so through partnerships with regional and national biotechnology or pharmaceutical companies.
- **Exploit the potential of our gene therapy platform to develop AAV-based gene therapies for additional orphan monogenic diseases and selected chronic and degenerative diseases.** We believe that gene therapy is well-suited as an approach for the treatment of monogenic diseases as only a single genetic defect needs to be addressed. We are initially focusing on orphan monogenic diseases that affect small patient populations because we expect to be able to complete clinical trials with relatively small numbers of patients and take advantage of the specialized regulatory approval processes for these rare conditions that exist in some countries. As a result, we anticipate that these programs may be more cost-effective to complete and have shorter timelines than are customary for other diseases and conditions. In addition to our programs in monogenic diseases, we plan to develop gene therapies for chronic and degenerative diseases that result from the body's inability to produce a necessary protein or enzyme and that affect larger populations, such as Parkinson's disease.
- **Leverage our competitive strengths to retain our position as a leading gene therapy company and to establish additional collaborations.** We believe our experience and expertise in gene therapy research and development and our proprietary manufacturing capabilities make us an attractive collaborator for academic research institutions and biotechnology and pharmaceutical companies seeking to advance their programs into larger, late-stage clinical trials that require commercial-scale manufacturing. We believe that these collaborations will enable us to gain access to early clinical programs and related

data, as well as promising transgenes and other intellectual property, with limited financial investment by us. We also believe that we can be a consolidator of gene therapy assets by entering into license and other arrangements with these types of entities.

- **Continue to invest in our technology platform and expand our modular capabilities.** We are continuously innovating, building and expanding our vector delivery and manufacturing technologies to further capitalize on the potential of gene therapy. We are currently focusing on developing:
  - next-generation AAV vectors with the potential for increased gene expression through improved cell-specific delivery and efficient release of DNA in the cell nucleus;
  - methods to allow successful re-administration in cases in which a one-time treatment may not be sufficient; and
  - methods for the successful control of gene expression following gene transfer, including the ability to terminate expression if needed.

## Product and Development Pipeline

In addition to Glybera, our development pipeline includes our internal program for hemophilia B, two collaborator-sponsored programs for monogenic diseases, one collaborator-sponsored program for a chronic degenerative disease and several programs in early preclinical development. The following chart provides summary information on the most advanced of these programs:

Product / Product Candidate	Vector	Gene	Indication	Collaborator	Development Stage				Comments
					Pre- Clinical	Phase I / II	Phase II / III	Approved	
Internal Programs									
Glybera (E.U.)	AAV1	Lipoprotein Lipase (LPL)	LPLD	Chiesi	EU Commercial launch planned first half of 2014				• Post-approval study initiation in first quarter of 2014
Glybera (U.S.)	AAV1	LPL	LPLD	-	IND filing planned in first half of 2014				• Met with FDA in August and December 2013 to discuss regulatory pathway
Glybera (Rest of World)	AAV1	LPL	LPLD	-	Targeting markets that recognize EU marketing authorization				• Discussions with potential marketing collaborators ongoing
AMT-060	AAV5	Human Factor IX (hFIX) <sup>(1)</sup>	Hemophilia B	Chiesi					• Phase I/II trial by St. Jude using AAV8 & uniQure's hFIX transgene is ongoing • uniQure Phase I/II planned to commence second half of 2014
Collaborator Sponsored Programs									
AMT-021	AAV5	Porphobilinogen Deaminase <sup>(1)</sup>	Acute Intermittent Porphyria (AIP)	Digna Biotech (Licensor: CIMA)					• Phase I clinical trial by Digna Biotech ongoing
AMT-110	AAV5	NaGLU	Sanfilippo B Syndrome	Institut Pasteur (Sponsor: AFM)	Phase I/II commenced in October 2013				• Phase I/II clinical trial by Institut Pasteur commenced in October 2013
AAV2 Delivering GDNF <sup>(1)</sup>	AAV2	GDNF <sup>(1,2)</sup>	Parkinson's Disease	UCSF (Funder & Sponsor: NIH)					• Phase I trial by UCSF / NIH using AAV2 & GDNF transgene ongoing

internal programs

collaborator sponsored programs

third party trials using a uniQure transgene

(1) hFIX, GDNF and PBGD transgenes have been exclusively licensed to uniQure.

(2) The trial commenced in May 2013; gene therapy was produced using mammalian-cell based process.

## Internal Program: Glybera

### Overview

Glybera is a gene therapy that is designed to restore the LPL enzyme activity required by tissues of the body to clear, or process, fat-carrying chylomicron particles that are formed in the intestine and transported via the lymphatic system to the blood after a fat-containing meal. The product consists of an engineered copy of the human LPL gene packaged with a tissue-specific promoter in a non-replicating AAV1 vector, which has a particular tropism for muscle cells. In order to improve activity, we use a naturally occurring variant of the LPL gene that has higher enzyme activity than the normal version of the gene that encodes the protein. We produce Glybera using our insect cell-based manufacturing process. Clinicians administer Glybera in a one-time series of up to 60 intramuscular injections in the legs. The patient is administered spinal anesthesia or deep sedation during the procedure. In addition, an immunosuppressive regimen is recommended from three days prior to and for 12 weeks following Glybera administration.

### LPLD Disease and Market Background

LPLD is a serious, debilitating disease caused by mutations in the LPL gene, resulting in significantly diminished or absent activity of the LPL protein. LPLD results in hyper-chylomicronemia, or dramatic and potentially life-threatening increases in the level of large fat-carrying particles, called chylomicrons, in the blood after eating. LPLD patients suffer from a wide range of serious disease manifestations. A prominent manifestation of the disease, resulting from elevated levels of chylomicrons in the blood, is acute and recurrent pancreatitis, which often leads to recurrent admission to hospitals and intensive care units, or ICUs. Chronic pancreatitis may also lead to pancreatic insufficiency that may result in decreased or absent production of digestive enzymes, increased risk of glucose intolerance and diabetes mellitus. In addition, the chronic dysregulation in lipid metabolism may lead to an increased risk of cardiovascular events. The most severe cases of acute pancreatitis are associated with an increased risk of death. In daily life, LPLD patients experience recurrent and chronic abdominal pain, eruptive xanthomas, or depositions of yellowish cholesterol-rich material in the skin, and neurological manifestations, which include headache, itching, tingling and burning sensations.

Women with LPLD experience additional complications. During pregnancy, natural increases in triglycerides may increase the risk of pancreatitis, which can put both the mother and the unborn child at considerable risk. Extreme dietary fat restriction to less than two grams per day during the second and third trimester with close monitoring of plasma triglyceride concentration may be required. Breastfeeding may not be possible beyond the first few days since the breast milk is unlikely to be nutritionally complete. The likelihood of gestational diabetes is increased in LPLD mothers. Furthermore, oral birth control and hormone replacement therapy are not advised since estrogen can cause dramatic increases in plasma triglycerides, which may lead to pancreatitis.

Prior to Glybera, there was no approved therapy for the treatment of LPLD. Clinicians advise LPLD patients to adhere to a strict diet restricting fat to less than 20% of daily calorie intake and to abstain from alcohol. Compliance with this dietary regimen is very difficult. Even with good compliance, the regimen is often ineffective in reducing hyper-chylomicronemia. LPLD patients therefore remain at increased risk for potentially lethal pancreatitis. These restrictions, as well as the need for frequent hospitalizations and the constant fear of encountering pancreatitis attacks, have a significant negative impact on the daily activity level of LPLD patients and on their quality of life.

The medical literature generally states that the prevalence of LPLD is approximately one person per million people. However, we believe that this prevalence number was not based on an epidemiological study, but rather was simply an estimate based on a non-systematic review of individual published case reports of patients with the disease. Historically, physicians have not routinely tested patients for LPLD as there was no reason to do so in light of the absence of any treatment options. In market research that we

commissioned from IMS Health, an international health information firm, key opinion leader physicians in the United States generally were of the view that LPLD may be significantly under-diagnosed.

We commissioned a third party study conducted in 2011 in Germany and the Netherlands of an experimental LPLD diagnostic test for LPLD. This unpublished study involved 314 patients with severe hypertriglyceridemia at 15 lipid centers. Severe hypertriglyceridemia is a highly abnormal elevated level of triglycerides in the blood and is commonly found in LPLD patients. In this study, eight of the patients tested had at least one known pathogenic mutation of the LPLD gene and a clinical manifestation of LPLD.

The number of likely LPLD patients as a percentage of the total patients in this study (8/314) was, therefore, 2.55%. In a 1982 study by Brunzell and Bierman published in *Medical Clinics of North America*, the authors estimated that there were approximately 180 persons per million in the United States with severe hypertriglyceridemia. On this basis, there would be approximately 4.6 persons with LPLD per million people ( $180 \times 2.55\%$ ). Because of the small number of patients in the study described above and the absence of other studies, we currently estimate the number of LPLD patients per million people to be in the range of three to six. This estimate is preliminary in nature, and we plan to conduct additional studies to establish a more precise estimate. Based on an article by Tremblay et al. published in the *Journal of Lipidology* in 2011, we further estimate that approximately 50% of persons with LPLD experience severe or multiple pancreatitis attacks.

### **Glybera Regulatory Status**

In October 2012, the European Commission granted a marketing authorization for Glybera under exceptional circumstances as a treatment for adult patients diagnosed with familial LPLD confirmed by genetic testing, detectable levels of LPL protein, and suffering from severe or multiple pancreatitis attacks despite dietary fat restrictions. The EMA ultimately assessed the combined quality, safety and efficacy data sets collected in the period from 2005 to 2011 using a totality of evidence approach. On this basis, the EMA considered clinical benefit to be sufficiently established to allow for positive benefit-risk estimation in an exceptional circumstance setting. Marketing authorization under exceptional circumstances in the European Union is available for products for which the target indications are so rare that comprehensive data on efficacy and safety cannot reasonably be expected to be available prior to commercial launch. In the European Union, we have been granted orphan drug exclusivity for Glybera for treatment of LPLD until October 2022, subject to the conditions applicable to orphan drug exclusivity. The FDA has also granted orphan drug designation to Glybera for treatment of LPLD.

When we began this clinical program in 2005, researchers had only a poor understanding of the link among the missing LPL enzyme activity, measurable metabolic lipid dysregulation and the actual disease manifestations of LPLD. Earlier research had suggested an association between higher than normal plasma triglyceride levels and pancreatitis, and we therefore believed at the time that triglycerides could provide a useful surrogate marker for a reduction in pancreatitis risk. Based on this hypothesis, initially we aimed to demonstrate a relevant and sustainable reduction in plasma triglycerides in our clinical trials. As the clinical development program progressed over the following years, however, we developed new mechanistic insights that led us to shift our focus from triglyceride levels to reductions in the level of newly formed chylomicrons after a meal as a relevant biological marker of LPL activity, and as such an appropriate surrogate marker for efficacy. During the clinical development program, we refined our working hypothesis and established a scientific rationale that links the expression of enzymatically active LPL and sustained improvement of such chylomicron metabolism after a meal with a reduced risk of pancreatitis attacks.

Within the EMA, the Committee for Human Medicinal Products, or CHMP, assesses drugs for human use. For gene and cell therapy drugs, the CHMP coordinates its assessment with the EMA's Committee for Advanced Therapies, or CAT, which is primarily responsible for the scientific evaluation of gene and cell therapies. The CAT provides a draft opinion to the CHMP on the quality, safety and efficacy of gene and cell therapies that are submitted for approval.

We initially submitted a marketing authorization application to the EMA in December 2009 and finally received marketing authorization in October 2012. During the review process, we answered, to the CHMP's and CAT's satisfaction, all of their questions regarding the safety of the vector and the manufacturing process, which had historically been matters of key concern for gene therapies. Nevertheless, in their initial decision in June 2011, both the CAT and the CHMP determined that the benefit-risk balance was negative for the treatment of all patients with LPLD. We requested a reexamination, and in October 2011, the CAT gave a positive opinion for the treatment of the subset of patients with LPLD suffering from severe or multiple pancreatitis attacks despite dietary fat restrictions. The CHMP, however, determined that there were not enough data points to show efficacy in a sufficient number of patients over a sufficiently long time period, and again recommended against approval at that time.

In January 2012, the European Commission recommended that the CHMP reassess its decision, requesting a further review for the proposed use of Glybera only in patients with severe or multiple pancreatitis attacks. In April 2012, the CHMP recommended against approval, but this recommendation was considered void for procedural reasons, and the CHMP then reassessed Glybera again for the proposed restricted population. In June 2012 the CAT gave a positive opinion and in July 2012 the CHMP recommended approval for the restricted population of adult patients diagnosed with familial LPLD confirmed by genetic testing, with detectable levels of LPL protein, and suffering from severe or multiple pancreatitis attacks despite dietary fat restrictions, subject to specified conditions, including additional post-marketing studies for efficacy. The CHMP recommendation was made by the majority of its members, with 17 members voting in favor and 15 dissenting. The European Commission granted this approval in October 2012.

Pursuant to the European Commission's decision to approve Glybera under exceptional circumstances, Glybera must be administered only under strict medical supervision. In addition, we must undertake an additional post-approval clinical trial and establish a patient registry, described below.

### **Post-EU Approval Program for Glybera**

**Post-Approval Clinical Trial.** We currently plan to enroll 12 patients with LPLD, along with eight healthy volunteers, in our planned post-approval clinical trial of Glybera. LPLD patients will receive a fixed dose of Glybera of  $1 \times 10^{12}$  genome copies per kilogram of body weight, which is the measure of the number of copies of the gene administered to the patient based on the patient's weight, in a single series of intramuscular injections. The LPLD patients will also receive an immunosuppressive regimen for a period beginning three days and ending 12 weeks after Glybera administration. We expect to enroll four LPLD patients per year and to complete the study by the middle of 2019, including a two-year follow up period. The primary objective of this post-approval study will be to investigate the effect of Glybera on chylomicron metabolism after a meal in LPLD patients both prior and after treatment with Glybera over a two-year period. We also intend to investigate:

- the clinical response of Glybera in LPLD patients using a range of parameters, including the incidence and severity of pancreatitis attacks, general LPLD disease manifestations and quality of life scales; and
- chylomicron metabolism after a meal in healthy volunteers.

The EMA has approved the protocol for this clinical trial. We currently anticipate that we will commence this clinical trial in the first quarter of 2014. We anticipate that we may seek to amend the protocol for this post-approval trial to address the requirements of the FDA, as described below.

**Planned LPLD Registry.** Another condition of the EMA approval of Glybera under exceptional circumstances is that we maintain a registry of LPLD patients. The objectives of the LPLD registry are to:

- collect, analyze and present available clinical safety and efficacy information in LPLD patients treated with Glybera over a 15-year period; and
- to collect natural history information in LPLD patients not treated with Glybera.



We intend to collect data during the course of treatment and at approximately annual intervals during the first two years post-treatment and every two to three years thereafter. The objectives will be to assess the:

- long-term safety of Glybera;
- long-term clinical response to Glybera; and
- epidemiology of LPLD and the demographics of LPLD patients.

The EMA has reviewed and approved the protocol for this patient registry. We anticipate that we will commence the patient registry in the first quarter of 2014. We are required to implement this registry prior to the commercial sale of Glybera.

We are also currently in the process of collecting additional long-term follow-up information in patients previously treated with Glybera, as an extension of the case note review described below, in order to further substantiate the data that we initially submitted to the EMA and that we intend to include in the data package we expect to submit to the FDA.

#### ***Planned United States Program for Glybera***

We met with the FDA in August and December 2013 to discuss the regulatory pathway in the United States for Glybera. In contrast with the European Union, the United States does not have a process to approve marketing of a drug under exceptional circumstances. In our meetings, the FDA advised that it would require data in addition to what we had submitted to obtain marketing approval for Glybera in the European Union and that it would not accept changes in chylomicron metabolism in isolation as a clinically meaningful biomarker for efficacy. The FDA recommended that we identify the clinical manifestations of LPLD for which Glybera might have the best prospects for demonstrating a meaningful impact in designing an adequate and appropriately controlled confirmatory trial. We may seek to amend the protocol for the European Union post-approval trial of Glybera described above so that such trial also could serve as such a confirmatory trial with a design that addresses the FDA requirements. In any event, we plan to file an IND with the FDA for Glybera in the first half of 2014 so that we can include United States LPLD patients in the post-approval trial. We also believe the patient registry described above that we are required to establish as part of our post-EU approval program will provide valuable data for the FDA to consider as part of the totality of our United States regulatory submissions. Our current expectation, subject to satisfactory completion of regulatory discussions with the FDA, is to have sufficient data from a confirmatory trial of Glybera and the patient registry to file a BLA for Glybera with the FDA in late 2016 or 2017.

#### ***Glybera Commercialization Plan***

We expect to launch Glybera commercially through our collaboration with Chiesi in selected countries in the European Union in the first half of 2014. We and Chiesi are working together through a joint commercialization committee to, among other things, plan a market roll-out strategy in the territory covered by the agreement, including developing a business model for the commercialization of a therapy administered in a one-time intervention. We and Chiesi are building new models for product pricing and reimbursement, expanding key opinion leader relationships, identifying centers of excellence, and developing physician and patient education and patient access programs.

***Pricing and Reimbursement in the European Union.*** To obtain payment coverage for Glybera from the relevant pricing and reimbursement agencies in countries in the European Union, Chiesi must generally submit price and reimbursement dossiers to the relevant bodies in each country. Chiesi is in discussions with these bodies in several countries, and expects to begin commercial sales during the first half of 2014. We expect that reference prices in the larger countries in the European Union will provide a basis for pricing discussions in other countries in the European Union. Pricing and reimbursement decisions are made on a country-by-country basis in the European Union and no country is under the obligation to follow another's pricing; however, prices in one country can influence the price level in other countries.



In developing our pricing strategy for Glybera we have commissioned third party research studies by Simon Kucher & Partners, a global consulting firm specializing in strategy, marketing, pricing and sales, on the pricing framework and are developing business models for gene therapies and orphan drugs in the markets we are targeting. In developing our pricing strategy, we and Chiesi considered current orphan disease treatments. We believe that Chiesi will seek a price for Glybera in the European Union reflecting the anticipated multi-year benefit of a one-time administration therapy and the unmet medical need of LPLD patients. We also believe that Glybera's effect in reducing pancreatitis attacks will result in a reduction of related hospitalizations and ICU stays, with attendant cost savings to the health care system. Based on the disease and therapy profiles, we believe that a one-time, up-front pricing model may be more in line with current analysis in pricing and reimbursement than an annuity model, which would provide for recurring periodic payments over a patient's lifetime. We therefore currently consider that a one-time price per patient treatment with Glybera to be the likely pricing model.

As an example of the pricing and reimbursement process, in Germany, the largest European Union market, commercial sales of Glybera in the out-patient setting could begin immediately after submitting the price and reimbursement dossier to the Gemeinsamer Bundesausschuss, or G-BA, even if the competent pricing and reimbursement bodies have not completed the benefit assessment and a price has not been agreed at that time. The G-BA decides on early benefit assessment of innovative pharmaceuticals. A different final price may take effect following the final price assessment, which may take up to 12 months following the initial sales of Glybera, and sales made thereafter would be made at that final price.

A further option for market access and sales of Glybera in Germany would be within the in-patient setting via a process known as Neue Untersuchungs- und Behandlungsmethoden, or NUB. Each hospital must separately apply for pricing and reimbursement levels for technologies that have recently been introduced in Germany. Such approvals can take significant time.

**Commercial Preparation and Roll-Out.** Chiesi plans to identify centers of excellence in each of the five largest European Union markets—France, Germany, Italy, Spain and the United Kingdom—where Glybera will be administered. Chiesi is developing a strategy to facilitate patient referrals to these centers, in part through broader educational efforts and outreach to relevant medical practitioners throughout the European Union. As part of this effort, we have established a publications library of clinical and non-clinical materials regarding Glybera and materials for key opinion leaders, as well as materials regarding LPLD and gene therapy generally.

If we obtain marketing approval for Glybera in the United States, we currently plan to commercialize Glybera ourselves. We have begun preliminary preparations for a potential launch in the United States, including commissioning a third party pricing and reimbursement study, and have conducted two market research studies directed at key opinion leaders. We have also initiated the development of a diagnostic referral program, engaged in key opinion leader and patient identification efforts and begun networking with key patient organizations in the United States.

#### ***Glybera Clinical Development to Date***

Our clinical development program for Glybera to date has consisted of three noncontrolled, prospective, open-label clinical trials in which we administered Glybera to a total of 27 LPLD patients. In addition, we carried out a retrospective case note review of 17 of the 27 patients to determine the impact of Glybera treatment on the frequency and severity of pancreatitis events. Our clinical development program for Glybera included trials with our AMT-011 product candidate, which was produced using our insect cell-based manufacturing process, as well as AMT-010, a predecessor product candidate produced using a mammalian cell-based manufacturing process.

### *Overall Results of Clinical Program*

In recommending approval of Glybera for a subset of LPLD patients under exceptional circumstances in July 2012, the CHMP accepted that the effect of Glybera on chylomicron metabolism could be considered a relevant biological marker of efficacy, although it noted that this had not been fully validated. The CHMP also recognized that, given the combination of the rarity of the disease and the varying levels of genetic penetration in LPLD patients, we were unable to provide comprehensive data on efficacy and safety under normal conditions of use prior to approval. The CHMP further noted the lack of consistency of the data on clinical benefit. Using a totality of the evidence approach, the CHMP assessed the quality, efficacy and safety of Glybera by combining the information from the different trials with individual patient profiles. On this basis, the following combined results assessed across the three clinical trials and the case note review from our clinical development program for Glybera provided the basis for the CHMP's positive recommendation to approve Glybera under exceptional circumstances:

- In one clinical trial, we observed a clear indication of a consistent and significant biological effect of Glybera on chylomicron metabolism after a meal, with significant improvement in chylomicron metabolism in all five patients seen at week 14 and all three patients seen at week 52 after a single treatment.
- In the clinical case note review involving a total of 17 patients, we observed a reduction in pancreatitis events and severity of attacks in nine of the 12 patients who had a history of pancreatitis. Although these observations were made in a small number of patients for varying pre-treatment observation periods, and subject to statistical limitations, they suggested that Glybera leads to a clinically relevant reduction of pancreatitis risk in nine of 12 patients with severe or multiple pancreatitis attacks. This was supported by the reduction in the total number of hospital admissions and ICU stays.
- On the basis of the clinical program and the case note review, a positive benefit/risk was considered shown in the subset of patients defined by the restricted indication proposed for Glybera in adult patients diagnosed with LPLD and suffering from severe or multiple pancreatitis attacks despite dietary fat restrictions. The indication was restricted to patients with detectable levels of LPL protein.
- A link between LPL expression, sustained improvement of chylomicron metabolism after a meal, both at 14 and 52 weeks following treatment, and a reduced risk of pancreatitis attacks was observed in two subjects who suffered from multiple recurrent pancreatitis and abdominal pain events before treatment. These findings, although highly limited by the limited number of observations, suggested a correlation between biochemical and clinical data in these two individual subjects. Sustained improvement in chylomicron metabolism after a meal in a third subject 52 weeks following treatment was also noted. Further, the data on LPL enzyme expression and function in injected and non-injected muscles, along with other relevant clinical and preclinical data provided indications of functional expression.

The following table provides key information about the clinical program that we have carried out to date for Glybera:

### Summary of Glybera Clinical Development Program

STUDY:	NO. OF PATIENTS	OBJECTIVES	DURATION OF FOLLOW-UP
<b>Retrospective Analysis:</b>			
Case Note Review AMT-011-03	17	<ul style="list-style-type: none"> <li>Effect on frequency and severity of pancreatitis in patients treated with Glybera in prior clinical trials</li> </ul>	Retrospective case note review of patients through 2010
<b>Clinical Trials:</b>			
Phase II/III trial AMT-011-02	5	<ul style="list-style-type: none"> <li>Effect on chylomicron metabolism at 14 and 52 weeks</li> <li>LPL activity at 3 months</li> <li>Safety</li> </ul>	1 year
Phase II/III trial AMT-011-01	14	<ul style="list-style-type: none"> <li>Safety</li> <li>Effect on triglyceride levels at 12 weeks</li> <li>LPL activity in the muscle at 6 months</li> </ul>	5 years
Phase I/II trial AMT-010-01	8	<ul style="list-style-type: none"> <li>Safety</li> <li>Effect on fasting triglyceride levels at 12 weeks</li> </ul>	5 years

**Safety.** In our clinical development program, Glybera demonstrated an acceptable overall safety profile. There were a total of 48 serious adverse events in these three clinical trials, only two of which were determined to be related to Glybera, a pulmonary embolism and fever. The most frequent adverse events were reactions associated with the injection procedure. These reactions were transient and mild to moderate. Further, immune responses to either the vector or the transgene were mild and there was no clinical evidence of long-term effects on muscle function, although muscle function was not formally tested. The shedding data illustrated that the vector was gradually eliminated from various bodily fluids with only low concentrations detected beyond 12 weeks following treatment.

#### AMT-011-03: Case Note Review

From January 2011 to March 2011, we conducted a review of hospital admission and discharge records for 17 patients previously treated with Glybera in clinical studies AMT-011-01 and AMT-011-02, in order to determine the frequency and severity of reported acute abdominal pancreatitis episodes. These data included:

- historic acute abdominal pain events;
- the development of chronic pancreatitis; and
- the development of pancreatic insufficiency.

In addition, we collected additional information regarding past medical history and prior hospital admissions of the patients.

We presented the data from individual patients as subject profiles to a panel of independent medical experts with expertise in the assessment and treatment of LPLD subjects and pancreatitis. This panel

evaluated these data using the Revised Atlanta Diagnostic Criteria, which are international consensus criteria commonly used to classify acute abdominal pain events as either "definite acute pancreatitis," "probable acute pancreatitis," "abdominal pain" or "other." The panel evaluated the number, frequency, and severity of episodes of pancreatitis and then the extent of LPLD disease progression. The panel identified 77 events in subjects treated with Glybera that conformed to the Atlanta Diagnostic Criteria for pancreatitis. Using retrospective control data, the analysis of pancreatitis events indicated that the frequency of acute abdominal events and pancreatitis in LPLD subjects decreased after the treatment with Glybera, but the data in isolation were not considered sufficiently strong to support a claim of a clinically relevant reduction in pancreatitis risk.

For this reason, in its assessment of the data from the AMT-011-03 case note review, the CHMP requested that data also be presented in the form of individual patient profiles in the 12 patients with the most severe manifestations of the disease, multiple recurrent pancreatitis and abdominal pain events, before treatment. The profiles indicated a reduction in pancreatitis events and severity of attacks in nine of these 12 patients, suggesting that Glybera leads to a clinically relevant reduction of pancreatitis risk in patients with severe or multiple pancreatitis attacks. This was supported by the reduction in hospital admissions and ICU stays.

#### *Phase II/III Clinical Trial (AMT-011-02)*

We initiated our second Phase II/III clinical trial of Glybera in Quebec, Canada in the first quarter of 2009. We describe this as both a Phase II and Phase III clinical trial because it was designed to support, if successful, an application for marketing approval of Glybera. We utilized our insect cell-based manufacturing process to manufacture the Glybera used in this trial. The purpose of this study was to understand the effect of Glybera on chylomicron metabolism and to evaluate and validate the use of a radiolabeled tracer to measure the appearance and removal of newly formed chylomicrons after eating as a relevant biological marker and primary endpoint instead of total plasma triglyceride levels. We treated five patients. Per the inclusion criteria, all trial participants:

- suffered from LPLD as confirmed by genetic testing;
- were on a low-fat diet with reduced LPL activity;
- had LPL activity 20% or less of normal levels;
- had LPL mass at least 5% above normal;
- had triglyceride levels of more than 10 millimoles per liter, or mmol/L, which is the level indicating increased risk of pancreatitis; and
- had a history of pancreatitis.

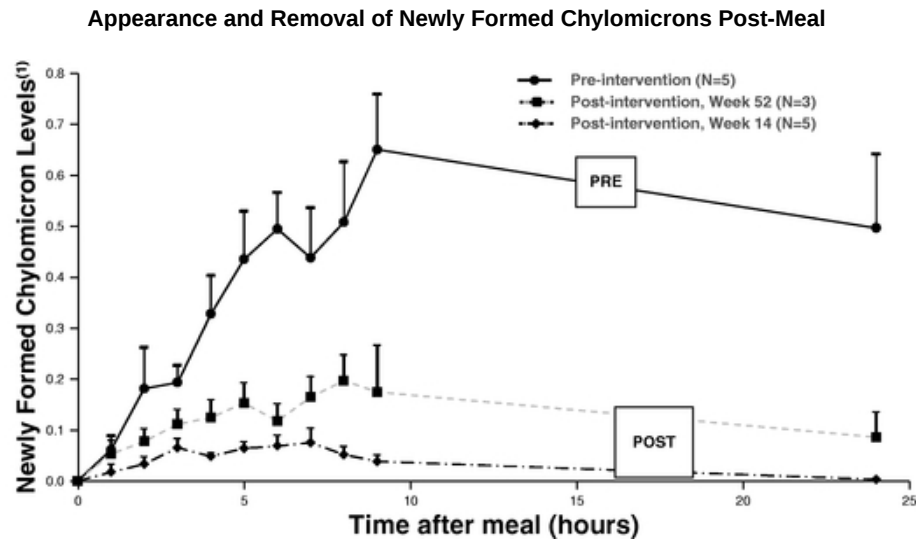
Each patient received one intramuscular dose of  $1 \times 10^{12}$  genetic components per kilogram of body weight, or gc/kg, which is the measure of the number of copies of the gene administered to the patient based on his weight as well as an immunosuppressant course of treatment to prevent immune responses to Glybera interfering with efficacy results.

The key results were as follows:

- in the five patients measured at 14 weeks, metabolism of chylomicrons newly formed after eating improved over baseline, with a reduction of an average of 79% at six hours after eating and an average of 93% at 24 hours after eating, compared with baseline; and
- in the three patients measured at 52 weeks, improvements in metabolism of chylomicrons newly formed after eating as compared with baseline persisted, with a reduction of an average of 50% at six hours after eating and an average of 68% at 24 hours after eating, compared with baseline.

The graph below depicts the appearance and removal of newly formed chylomicrons in the blood over a 24-hour period after a standardized meal, through use of a radiolabeled tracer. Patients were observed prior to treatment with Glybera, and at 14 weeks and 52 weeks following treatment. In LPLD patients only very limited and slow metabolism of chylomicrons occurs during a 24-hour period. The top line in the graph

represents the pretreatment condition and depicts mean levels of newly formed chylomicrons in the five patients prior to treatment with Glybera. We observed a consistent and significant improvement in the appearance and removal of newly formed chylomicrons from the blood in all five patients measured at week 14 after treatment, represented by the bottom line in the graph, and all three patients measured at week 52 after treatment, represented by the middle line in the graph. We believe that the improvement in newly formed chylomicron metabolism reflects an increase in LPL activity post treatment with Glybera.



<sup>(1)</sup> Depiction of mean levels of newly formed chylomicrons using a radiolabeled tracer measured by tritium activity (in centimorgans). Participants consumed a standardized meal containing tritium-marked particles, which were measured in newly formed chylomicrons in the 24-hour period after the meal.

In addition, in muscle biopsies in three out of five patients, a one-time administration of Glybera led to sustained expression of the LPL gene and biological activity of the protein in muscle. The muscle biopsy data obtained post-intervention, together with the chylomicron data, suggest that Glybera resulted in enzymatically active LPL expression in these patients, and that this restored LPL actively was able to exert an effect on lipid metabolism despite plasma triglyceride levels typically returning to baseline by this time point.

#### *Phase II/III Clinical Trial (AMT-011-01)*

Our first Phase II/III, clinical trial of Glybera was a dose escalation trial, which we initiated in Québec, Canada, in the third quarter of 2007. We utilized our insect cell-based manufacturing process to produce the Glybera used in this clinical trial.

We treated a total of 14 LPLD patients under the same principal inclusion criteria as in our 2009 Phase II/III clinical trial described above. Prior to the clinical trial, subjects participated in an observational study to establish baseline data. We divided patients into three cohorts for treatment with a single-dose intramuscular injection. One cohort of two patients and one cohort of four patients each received an intermediate dose of  $3 \times 10^{11}$  gc/kg, and the third cohort of eight patients received a lower dose of  $1 \times 10^{12}$  gc/kg. The second higher-dose cohort and the third cohort were also given an immunosuppressant course of treatment to prevent immune responses to Glybera interfering with efficacy results, based on our observations in our Phase I/II clinical trial. We established the cohort without this course of immunosuppressant as a bridge from the results of our Phase I/II clinical trial.

The key results relating to the primary endpoints were as follows:

- seven of the 14 patients, or 50%, reached the primary efficacy endpoint of a 40% reduction in median triglyceride levels during the period three to 12 weeks after treatment, including five of the eight patients, or 62.5%, in the highest dosing cohort; and

The results relating to the secondary endpoints were as follows:

- four patients met the secondary efficacy endpoint of a median fasting plasma triglyceride level of less than or equal to 10 mmol/L between three and 12 weeks post-treatment; and
- initial reductions in plasma triglyceride levels were statistically significant, but at 26 weeks post-treatment plasma triglyceride levels had returned to baseline.

In addition, we observed the following:

- lipid uptake in muscle cells was evident in biopsies in all seven participants from whom biopsies were obtained; and
- evidence supporting changes in lipoprotein distribution, which supported the rationale for the subsequent trial.

As described above, during the course of our clinical development program for Glybera we developed new mechanistic insights that led us to shift our focus from triglyceride levels to reductions in chylomicron levels after a meal as a biological marker of LPL activity.

#### *Phase I/II Clinical Trial (AMT-010-01)*

Our initial Phase I/II clinical trial of Glybera was a dose escalation trial that we conducted at the Academic Medical Center, University of Amsterdam, Netherlands, in 2005. Unlike our later trials, in which we used Glybera that we produced with our insect cell-based manufacturing process, in this trial we used Glybera that we produced in a mammalian cell-based manufacturing process. We treated eight LPLD patients under the same inclusion criteria as in our 2009 Phase II/III clinical trial described above, except that we did not require a history of pancreatitis. Prior to the clinical trial, subjects participated in an observational study during which they maintained a low-fat diet in order to establish baseline triglyceride levels.

The key result relating to the primary endpoint was as follows:

- at 12 weeks after treatment, all patients showed a reduction in median triglyceride levels from baseline, and for three of them the reduction was greater than 40%.

The results relating to the secondary endpoints were:

- muscle biopsies 10 to 36 weeks after treatment in seven of the eight trial participants suggested that administration of Glybera led to long-term biological activity and expression of protein in the injected muscle; and
- we observed an immune response to AAV and determined that we should administer an immunosuppressant regimen in future trials.

From the point 12 weeks post-treatment to the long-term follow-up period at two to three years post-treatment, median triglyceride levels increased to around or above baseline, which we initially interpreted as a potential indication of transient efficacy.

## Internal Program: AMT-060 for Hemophilia B

### *Hemophilia B Disease and Market Background*

Hemophilia B is a serious rare inherited disease in males characterized by insufficient blood clotting. The condition can lead to repeated and sometimes life-threatening episodes of external and internal bleeding following accidental trauma or medical interventions. The episodes can cause long-term damage, for example to the joints, and can be fatal if they occur in the brain. The deficient blood clotting results from the lack of functional human Factor IX, or hFIX, a blood clotting factor, as a result of mutations in the relevant gene. The presence of hFIX at greater than 1% of normal levels has a therapeutic effect in promoting clotting. The current standard of care for hemophilia B is prophylactic or on-demand protein replacement therapy, in which frequent intravenous administrations of plasma-derived or recombinant hFIX are required to stop or prevent bleeding. Protein replacement therapy is expensive, often costing approximately \$220,000 to \$340,000 per patient per year in the United States. Such therapy is also burdensome and does not completely prevent bleeding.

Hemophilia B affects approximately 20 persons per million males in Europe, and 28 persons per million males in the United States, according to the World Federation of Hemophilia. Approximately 60% of individuals with the disease have severe hemophilia, according to the National Hemophilia Foundation, characterized by functional hFIX levels that are less than 1% of normal; 15% of the hemophilia population have moderately severe disease, with 1 to 5% of normal levels; and the remainder have mild disease, with 5 to 50% of normal levels. Based on these estimates we believe that the approximately 60 to 70% of the worldwide patient population with severe to moderate disease would be eligible for treatment with gene therapy.

### *Overview of AMT-060*

The goal of our AMT-060 program is to develop a gene therapy for hemophilia B that restores blood clotting on a long-term and potentially curative basis through the delivery of the functional gene for hFIX into the patient's liver cells. In collaboration with Chiesi, we are developing AMT-060 as a gene therapy consisting of an AAV5 vector carrying an hFIX gene that we have exclusively licensed from St. Jude, in which we have altered the codons to maximize expression, together with the insertion of a liver-specific promoter, LP1. We produce this vector with our insect cell-based manufacturing process. We are designing this therapy for systemic administration through intravenous infusion in a single treatment. We are currently preparing for a Phase I/II clinical trial with this product candidate, which we expect to commence in the second half of 2014. Our collaborator St. Jude is currently conducting a Phase I/II clinical trial in this indication with a gene therapy consisting of an AAV8 vector carrying the same therapeutic gene and produced in a third party mammalian cell-based manufacturing process. We have entered into a co-development agreement with Chiesi for the development and commercialization of AMT-060 in the European Union and other specified countries.

### *Planned Phase I/II Clinical Trials*

We are currently planning to initiate a Phase I/II clinical trial of our AMT-060 product candidate in the second half of 2014 under our co-development agreement with Chiesi. We intend to file an IND and IMPD in order to initiate clinical development of AMT-060 and anticipate that the trial sites will be in Europe. We are designing this clinical trial as a multicenter, open label, prospective, interventional, single dose, dose escalation study to investigate the safety and tolerability of AMT-060 in patients with severe hemophilia B. We currently expect to recruit 13 to 16 male patients between the ages of 18 and 35 with severe, genetically confirmed hemophilia B. The primary objective of this clinical trial will be to assess the safety of systemic administration and determine the maximum tolerated doses. We expect that the secondary objectives will include:

- determining the appropriate dose required to achieve delivery of the transgene resulting in stable expression of hFIX at or above 3% of normal;

- assessing the immune response to the hFIX transgene and to the AAV5 capsid proteins, as well as viral shedding; and
- assessing the need for concomitant hFIX treatment.

The draft protocol calls for initial patient follow-up to last for six months as part of the trial. We then plan to follow patients for safety and therapeutic response at intervals of 24 weeks for three years. We expect data from this clinical trial to be available in the second half of 2015.

#### *Preclinical Program with AMT-060*

We are currently conducting a number of preclinical safety and toxicology studies to support our development program for AMT-060, including studies in mice and non-human primates to measure pharmacokinetics, toxicity, shedding patterns, persistence in semen and risk of germline transmission, and carcinogenicity. We expect to complete these safety and toxicology studies by the end of 2013. We need to successfully complete these preclinical studies in order to file for regulatory clearance to initiate our planned Phase I/II clinical trial of AMT-060.

The principal results of our preclinical tests to date are as follows:

- In wild-type mice, intravenous administration of AMT-060 resulted in dose-dependent levels of hFIX levels in plasma. hFIX levels amounted to up to 11% of those in normal human plasma four weeks after infusion, indicating that AMT-060 produced in our insect-cell manufacturing process is biologically active.
- In Rhesus monkeys dosed at one dose level with a single treatment of AMT-060 by intravenous infusion, hFIX levels peaked to 7% to 16% of normal human levels one week after infusion, and stabilized at 5% to 10% of normal human levels two weeks after infusion until sacrifice at 12 weeks after dosing. These kinetics are in accordance with those we and others observed in previous studies, indicating that intravenous administration of AMT-060 produced in our insect cell-based manufacturing process results in a level of hFIX in plasma that is similar to that produced using AAV5 and AAV8 vectors produced in mammalian cells.
- Cynomolgus monkeys dosed at four dose levels with a single treatment of AMT-060 by intravenous infusion showed a linear dose response in relation to hFIX levels. At the top dose, expression levels plateaued at 7%, although the data showed significant variability among subjects. Monitoring over the six months following dosing demonstrated the treatment was well tolerated and safe.
- In mice studies, post-mortem tests showed homogeneous delivery of the vector DNA and transgene expression in the liver. We observed no signs of adverse reactions. Infusion was associated with slight and transient effects in plasma chemistry shortly after dosing, such as a brief increase of liver enzyme activity levels, consistent with the infusion of a viral protein. Necropsy revealed no significant macroscopic or microscopic abnormalities. Overall, administration of AMT-060 in mice resulted in therapeutically relevant hFIX levels and was well tolerated.

#### *Phase I/II Clinical Trial of AAV8-based hFIX Gene Therapy by St. Jude*

St. Jude is conducting a Phase I/II open label, dose escalation clinical trial of an hFIX gene therapy in adults with severe hemophilia B. The St. Jude gene therapy consists of the same therapeutic gene cassette we have exclusively licensed, delivered in an AAV8 vector. St. Jude produced the vector in human embryonic kidney cells. The principal investigators of the trial reported interim data from the trial in the *New England Journal of Medicine*, the NEJM, in December 2011. The information in this prospectus about this Phase I/II clinical trial is derived from the NEJM article. We understand from St. Jude that the final data from this clinical trial are expected to be released in the second half of 2015.

This dose escalation trial initially enrolled six male adults suffering from severe hemophilia B, with three dose cohorts of two patients each. The gene therapy was administered in a single dose by infusion into a



peripheral vein. St. Jude followed the participants for six to 16 months after treatment by way of twice weekly clinical evaluations.

The interim data indicated that the administration of the St. Jude gene therapy did not result in acute or long-lasting toxicity in patients with severe hemophilia B. Further, the interim data indicated the following:

- the high dose cohort achieved a stable expression of hFIX at or above 3% of the normal levels while the low and intermediate cohorts did not;
- St. Jude did not detect any neutralizing antibodies and the immune response was consistent with a primary immune response to AAV8; and
- St. Jude's did not detect T-cell, or white blood cell, mediated immune responses to hFIX.

St. Jude observed AAV-mediated expression of hFIX at 2% to 11% of normal levels in all patients. Four of the six patients discontinued prophylactic hFIX protein replacement therapy and remained free of bleeding even during activities that had previously led to hemorrhaging. Of the two participants who received the highest dose, one had a transient, asymptomatic elevation of serum aminotransferase levels, which was associated with the detection of AAV8-capsid-specific T cells in the peripheral blood, and the other had a slight increase in liver-enzyme levels, the cause of which was less clear. St. Jude administered a short course of glucocorticoid therapy to those two patients to reduce liver inflammation in an effort to maintain hFIX levels in the range of 3% to 11% of normal values.

Patients experienced a total of three adverse events. Two patients developed anemia after treatment and a third patient had a transient period of below-normal heart rate. No serious adverse events were reported.

We understand from public presentations by the principal investigators for this trial that two additional patients at the highest dose level in this clinical trial have now also demonstrated such long-term expression.

We believe that these interim results constitute proof of concept of the use of this therapeutic gene in treating hemophilia B, which may reduce the risks involved in the development of AMT-060.

### **Collaborator-Sponsored Programs**

We are also collaborating with third parties that are sponsoring early-stage clinical trials of gene therapy product candidates to which we hold specified rights. We believe that this approach enables us to cost-effectively obtain access to preclinical and early-stage clinical results without expending significant resources of our own. As described below, some of these programs utilize clinical materials that we have manufactured as part of our collaborations or gene cassettes that we have licensed. We generally have the rights to the data generated in these collaborator-sponsored clinical development programs, but do not control their design or timing. If we decide to progress any of these programs internally, we may need to develop or in-license additional technology. The most advanced of these programs are the following:

### ***Acute Intermittent Porphyria***

#### ***AIP Disease and Market Background***

AIP is a rare metabolic liver disorder resulting from mutations in the PBGD gene. This gene encodes for the enzyme porphobilinogen deaminase, a liver protein necessary for the production of heme, which is a component of hemoglobin and other blood proteins. Insufficient activity of this protein leads to an accumulation of toxic metabolites, resulting in a wide variety of serious clinical problems, including acute, severe abdominal pain, muscular weakness and an array of neurologic manifestations, including psychiatric episodes, seizures and coma. In the majority of cases, precipitating factors, such as hormonal fluctuations, infections, drugs and dietary changes, trigger attacks. Long-term consequences may include irreversible nerve damage, liver cancer and kidney failure. Patients with AIP experience regular hospitalizations and extremely poor quality of life. Acute attacks can be life-threatening. Current therapies include intravenous

administration of heme and carbohydrate loading, which aim to treat the symptoms only and do not prevent attacks. In some cases, AIP patients require liver transplants.

#### *Overview of AMT-021*

We and our collaborator Digna Biotech are developing AMT-021 as a gene therapy to provide long-term normalization of the PBGD protein in order to prevent acute AIP attacks and their complications. The AMT-021 gene cassette contains the PBGD gene, which we exclusively license from CIMA, in which we have altered the codons to maximize expression, together with Alb-hAAT, a liver-specific promoter. We package this gene cassette in our AAV5 vector, which we believe has a tropism for liver cells. We produce AMT-021 using our insect cell-based manufacturing process. AMT-021 is administered through a single intravenous infusion to a peripheral vein. We are a member of the AIPGENE consortium in Europe, through which Digna Biotech, a consortium member, is currently conducting a Phase I clinical trial of AMT-021.

#### *Phase I Clinical Trial Sponsored by Digna Biotech*

Digna Biotech commenced a multicenter, open label, prospective, interventional, single dose, dose escalation Phase I clinical trial in December 2012 to investigate the safety and tolerability of AMT-021 in eight patients with severe AIP. Digna Biotech is conducting this clinical trial at two sites in Spain. There are four dosing cohorts in the trial, with two patients per cohort. All patients have been dosed. Digna Biotech will monitor all patients for one year following treatment. Digna Biotech has also completed a prospective pre-treatment observational study of the eight patients enrolled in this clinical trial to assess the evolution of disease-related clinical and laboratory parameters over time and to characterize aspects of disease management, such as AIP-related hospitalization.

The primary objective of this Phase I clinical trial is to assess the safety of systemic administration and to determine the maximum tolerated dose of AMT-021. Secondary objectives include measuring urinary levels of toxic metabolites to determine whether these metabolites can be used as a biomarker of potential treatment effect.

Digna has advised us that through November 30, 2013, there was one serious adverse event in this trial that was determined by the investigator not to be treatment-related. Digna further reported that there were no treatment-related adverse events or liver events related to AMT-021. Digna does not plan to report clinical outcomes data from this Phase I clinical trial until the second half of 2014. However, to date, Digna has not observed a reduction in the urinary levels of toxic metabolites in trial participants that potentially could serve as a surrogate marker for efficacy. We believe that this result may suggest that a relatively high level of transgene expression will be required for a gene therapy to provide a clinical benefit in this indication. This contrasts with an indication such as hemophilia, in which the near or total absence of a protein in the patient means that a relatively low level of gene expression may result in a clinical benefit. In light of the absence of dose-limiting toxicities in the ongoing Phase I clinical trial, upon receipt of the final study data, we plan to consider continuing the trial at higher dose levels or initiating a new clinical trial with a new vector that we are developing that may provide increased potency. Under our consortium agreement with Digna Biotech and the other consortium members, following completion of this Phase I trial we have an exclusive right to use all data related to the program.

#### *Preclinical Program*

In preclinical tests by Digna Biotech, AMT-021 resulted in normalization of the PBGD protein in a mouse model of AIP. AMT-021 completely prevented the occurrence of AIP-related attacks and significantly ameliorated the neuropathy that develops in untreated mice. In these preclinical tests, AMT-021 also demonstrated a good safety profile. Key findings from these mouse studies include long-term therapeutic efficacy indicated by:

- the metabolic correction of the hepatic PBGD enzyme activity;
- improvement of motor coordination;

- clearance of AMT-021 from the blood and urine, but not the liver, by 30 days after administration; and
- expression of PBGD in the liver of mice for more than one year.

In addition, in normal non-human primates treated with AMT-021, PBGD enzymatic activity increased by a factor of two in males and by a factor of between three and five in females compared with endogenous levels.

### **AMT-110 for Sanfilippo B Syndrome**

#### *Sanfilippo B Syndrome Disease and Market Background*

Sanfilippo B syndrome, or mucopolysaccharidosis type III (MPSIII), is a rare lysosomal storage disease, or LSD, that results in serious brain degeneration in children, and is generally fatal. In this condition, a defect in the  $\alpha$ -N-acetylglucosaminidase, or NaGLU, gene results in the accumulation of partially degraded oligosaccharides, or carbohydrates, of heparan sulfate, which are molecules that regulate various developmental processes. NaGlu is necessary for the degradation of heparan sulphate. The clinical manifestations are mainly neurological, with early symptoms observed during the first five years of life, leading to progressive deterioration of cognitive abilities. Affected children require specialist care between ages two and six and progressively develop profound mental retardation with severe muscle problems. Death occurs at the median age of 15. No treatment for Sanfilippo B is currently available.

#### *Overview of AMT-110*

We and our collaborator Institut Pasteur are developing AMT-110 as a gene therapy for Sanfilippo B syndrome. The gene cassette contains the NaGLU gene and is packaged in an AAV5 vector, which we believe has a tropism for neurons. We produce AMT-110 using our insect cell-based manufacturing process.

We believe that if the results of this clinical trial are positive, it will constitute proof of concept of the administration to the brain of a gene therapy for lysosomal storage diseases.

#### *Phase I/II Clinical Trial with AMT-110 Sponsored by Institut Pasteur*

Our collaborator Institut Pasteur commenced a Phase I/II open label trial of intra-cerebral administration of AMT-110 for the treatment of children with Sanfilippo B syndrome in October 2013. We understand from Institut Pasteur that final data are expected in the first half of 2015. This Phase I/II clinical trial is being conducted in Paris, France, and is scheduled to run over an eight- to 12-month period, with a follow-up period of one year for each patient. Pursuant to our collaboration agreement with Institut Pasteur, we have manufactured the clinical material that Institut Pasteur is using in this trial.

The protocol for this single-dose Phase I/II clinical trial calls for the inclusion of four Sanfilippo B syndrome patients between the ages of 18 months and five years with NaGLU levels less than 10% of those found in the general population. Patients will receive an immunosuppressant course of treatment prior to administration of the therapy, to prevent an immune response to either the AAV vector capsid or the expressed protein. The primary objective is to evaluate the clinical, radiological and biological safety of the proposed treatment. The secondary objective is to collect data to define exploratory tests that could inform further clinical studies.

#### *Preclinical Development of AMT-110 by Institut Pasteur*

Institut Pasteur has conducted preclinical animal tests of AMT-110. Key findings of these studies include the following:

- rodents displayed no signs of toxicity at seven days, three months or six months after treatment despite administration of up to 37 times the level of dosage required for human patients;

- biodistribution studies in rodents indicated no differences between those following an immunosuppressant treatment course and those that were not, and shedding from major organs over time; and
- biodistribution studies in canine subjects indicated that the vector was absent in major organs approximately four months after administration.

## **AAV2/GDNF for Parkinson's Disease**

### *Disease and Market Background*

Parkinson's disease is a progressive neurodegenerative disorder that affects motor skills, speech and other neurological functions. The symptoms of Parkinson's disease result from degeneration and death of nerve cells in the putamen, a part of the brain that produces dopamine, which is a chemical that sends messages in the brain to coordinate and control muscular action and movements, and other neuro-transmitters. Progressive loss of nigral dopaminergic neurons, the pathological hallmark of Parkinson's disease, results in progressive neurologic dysfunction and death. There is currently no cure for Parkinson's disease. Medications or surgery can provide symptomatic relief, but they do not affect the degenerative process. In addition, the efficacy of these therapies declines over time, and they can result in significant side effects and co-morbidities, such as depression and a movement disorder called dyskinesias. The most widely used treatment is L-dopa in various forms, which is converted to dopamine in the central nervous system.

GDNF stimulates the production of dopamine in the putamen and prevents further degeneration of dopaminergic neurons in preclinical models. A series of preclinical and clinical studies by third parties involving the infusion of GDNF protein into the brain have shown potential benefit in treating Parkinson's disease. Results from these early clinical trials underscore the need for a clinical approach that can accurately introduce appropriate levels of GDNF to the intended sites in the brain where the dopaminergic neurons and their terminals reside.

### *Overview of AAV2/GDNF*

We and our collaborator UCSF are developing a gene therapy for Parkinson's disease. As described below, UCSF is collaborating with the NIH to conduct a clinical trial of a gene therapy consisting of an AAV2 vector carrying the GDNF gene we have exclusively licensed in the gene therapy field from Amgen, manufactured by a third party using a mammalian cell-based process. If we progress our AMT-090 program, we would transition this product candidate to our insect cell-based manufacturing process.

### *Phase I Clinical Trial Sponsored by the NIH*

Our collaborator UCSF is working with the NIH to conduct a Phase I clinical trial of a gene therapy for Parkinson's disease consisting of an AAV2-based vector carrying the GDNF gene we have exclusively licensed, produced in a third party mammalian cell-based manufacturing process. This trial is sponsored and funded by the NIH and will involve 24 patients. The aim of this clinical trial is to introduce the GDNF gene to provide a consistent supply of GDNF to the relevant areas of the brain. In this clinical trial, the NIH is using convection enhanced delivery with the goal of achieving more precisely targeted administration than the methods used in early approaches, which may result in improved efficacy. Convection-enhanced delivery involves MRI-guided placement of one or more catheters directly into the brain to provide distribution of the therapeutic agent to a larger volume of the brain tissue, provide higher drug concentrations directly to the tissue and to use molecules that do not normally cross the blood-brain barrier. We have the right to acquire all of UCSF's data from this clinical trial.

### **Potential Additional Pipeline Programs**

We are also conducting early-stage discovery and preclinical research, often in collaboration with academic research institutions, into a number of other potential applications of our technologies. Our principal near-term research focus is on diseases originating in the liver and the CNS. We believe that the liver is a promising target for gene therapies because we can both target liver-specific diseases and also use the liver to secrete proteins into the blood to have a systemic patient benefit. We believe that the CNS is also an attractive target organ for gene therapy as monogenic diseases that affect the brain are often poorly served by existing treatments, such as enzyme replacement therapies, which are not able to cross the blood-brain barrier following administration into the blood. Moreover, continual direct administration of proteins into the brain is practically difficult. Our AAV5-based vectors have a particular tropism for both the liver and CNS. We also plan to develop other AAV serotypes as appropriate to target specific indications.

We choose potential additional indications to develop independently or in conjunction with a collaborator, by applying the following criteria:

- we seek indications in which gene delivery would be expected to result in gene expression in the substantial proportion of cells of a target organ or tissue such that the symptoms of the underlying disease would be expected to be addressed;
- we seek indications in which a locally secreted protein would be expected to have systemic clinical benefit (in effect, the target organ is used as a protein factory) which may not necessarily require expression in a substantial proportion of the cells of the target organ or tissue;
- we seek indications for which relatively low restored protein expression levels as compared to normal would be expected to have therapeutic benefit;
- we target diseases that have sufficient prevalence to allow clinical development to be possible and for there to either be a viable commercial market in the indication or the indication provides proof of concept for related diseases;
- we look to select indications for which there is a robust and available animal model for preclinical testing;
- we seek indications in which the disease is sufficiently well-characterized such that it is reasonable to expect that if effective delivery of the relevant transgene is achieved, clinical efficacy should result; and
- we prioritize indications for which markers of biological activity are available that may permit assessment of benefit in early clinical studies.

Based on these criteria, we have prioritized approximately ten indications for preclinical development. We may seek to advance these programs independently or alternatively with collaborators who are already working in the relevant disease area and who may have already conducted preclinical or clinical studies.

Our current preclinical research and discovery programs include those described below:

#### *Liver Application:*

- **Hemophilia A.** Hemophilia A is an X-linked recessive genetic bleeding disorder. The disease results from the production of dysfunctional factor VIII protein or by production of an insufficient amount of factor VIII. Hemophilia A patients suffer from spontaneous bleeding into the large joints and soft tissue, and are at risk of intracranial hemorrhage. Even a modest 1% increase of the protein levels can markedly reduce spontaneous bleedings. We are developing an AAV5-based vector carrying the human factor VIII gene. The challenge for factor VIII development is in packaging the relevant gene, which is larger than the packaging capacity of the AAV vector. We believe we have successfully overcome this challenge by packaging the two different and complementary ends of a factor VIII DNA strand into different vectors for delivery to the cells of interest, where they recombine into a complete expression cassette. We have shown proof-of concept by tail vein injection of AAV5-factor VIII in

mice, which resulted in delivery of the transgene to liver cells and production of active factor VIII by the liver.

In addition, we are seeking to develop next-generation vectors with increased potency to target liver indications in which high relative percentage increases in the secretion of a protein above the disease state would be required for therapeutic benefit. One approach we are using is directed evolution, which involves a vector selection process in which libraries of mutant variants are screened for optimal properties.

#### *CNS Applications:*

- ***Lysosomal storage diseases.*** As noted above, we believe that if the results of Institut Pasteur's Phase I/II clinical trial in Sanfilippo B syndrome are positive, it will constitute proof of concept of the administration of a gene therapy for lysosomal storage diseases to the brain. In such event, we believe that we may be able to apply this approach to develop gene therapies with the goal of addressing a number of the more than 30 lysosomal storage diseases that have CNS-specific disease manifestations and for which no treatment is currently available. We are conducting preclinical research to advance the application of our technologies in this area. For example, we have shown in preclinical models widespread distribution of AAV5 in the brain when administered via the cerebral spinal fluid.
- ***Applications of GDNF.*** We are using our academic relationships to test proof of concept of the GDNF gene that we have exclusively licensed in the field of gene therapy in animals to extend the use of this potent neurotrophic factor, including potentially for the treatment of multiple systems atrophy, amyotrophic lateral sclerosis and hearing loss.

We also have ongoing research programs in the areas of gene expression control, re-administration protocols and nuclear targeting, to further increase expression levels and safety margin. We are also conducting research into potential applications of our technology in transcription silencing, also called post transcriptional gene silencing, which is a biological process in which RNA molecules inhibit gene expression, typically by causing the destruction of specific miRNA molecules.

#### **Intellectual Property**

##### ***Introduction***

We strive to protect the proprietary technologies that we believe are important to our business, including seeking and maintaining patent protection in the United States, Europe and other countries for novel components of gene therapies, the chemistries and processes for manufacturing these gene therapies, the use of these components in gene therapies, and other inventions and related technology that are important to our business, such as those relating to our technology platform. We also rely on trade secrets and careful monitoring of our proprietary information to protect aspects of our business that are not amenable to, or that we do not consider appropriate for, patent protection.

Our success will depend significantly on our ability to obtain and maintain patent and other proprietary protection for commercially important technology, inventions and know-how related to our business, defend and enforce our patents, maintain our licenses to use intellectual property owned by third parties, preserve the confidentiality of our trade secrets and operate without infringing the valid and enforceable patents and other proprietary rights of third parties. We also rely on know-how, continuing technological innovation and in-licensing opportunities to develop, strengthen, and maintain our proprietary position in the field of AAV-based gene therapies.

We also are heavily dependent on the patented or proprietary technology of third parties to develop and commercialize our products. We must obtain licenses from such third parties on commercially reasonable terms, or our business could be harmed, possibly materially. For example, we license from third parties essential parts of the therapeutic gene cassette used in Glybera and our other gene therapies, as well as the

principal AAV vectors we use and key elements of our manufacturing process. We anticipate that we will require additional licenses in the future.

Because most patent applications throughout the world are confidential for 18 months after the earliest claimed priority date, and since the publication of discoveries in the scientific and patent literature often lags behind actual discoveries, we cannot be certain that we were the first to invent or file applications for the inventions covered by our pending patent applications. Moreover, we may have to participate in post-grant proceedings in the patent offices of the United States or foreign jurisdictions, such as oppositions, reexaminations or interferences, in which the patentability or priority of our inventions are challenged. Such proceedings could result in substantial cost, even if the eventual outcome is favorable to us.

Our intellectual property portfolio consists of owned and in-licensed patents, licenses, trademarks, trade secrets and other intellectual property rights.

### ***Patent Portfolio***

Our gene therapy programs are protected by patents and patent applications directed to various aspects of our technology. For example, our gene therapy programs are protected by patents and patent applications with composition-of-matter or method of use claims that cover the therapeutic gene, the promoter, the viral vector capsid or other specific parts of these technologies. We also seek protection of core aspects of our manufacturing process, particularly regarding our baculovirus expression system for AAV vectors in insect cells. In addition, we have filed manufacturing patent applications with claims directed to alternative compositions-of-matter and manufacturing processes to seek better protection from competitors.

Our principal operations are currently in Europe and we are in the process of building out a new manufacturing facility in the United States. We file the initial patent applications for our commercially important technologies in both Europe and the United States. For the same technologies, we typically file international patent applications under the Patent Cooperation Treaty, or PCT, within a year. We also may seek, usually on a case-by-case basis, local patent protection in Canada, Australia, Japan, China, India, Israel, South Africa, New Zealand, South Korea and Eurasia, as well as South American jurisdictions such as Brazil and Mexico.

As of the date of this prospectus, our patent portfolio includes the following patent families:

- 13 patent families that we own;
- 8 patent families that we exclusively in-license; and
- 6 patent families that we non-exclusively in-license.

The geographic breakdown of our owned patent portfolio is as follows:

- 2 issued United States patents;
- 2 granted European Patent Office patents;
- 1 pending PCT patent application;
- 7 pending United States patent applications;
- 8 pending European Patent Office patent applications; and
- 57 pending patent applications in other jurisdictions.

The patent portfolios for our manufacturing platform and most advanced programs are summarized below.

### ***NIH Patents***

Our manufacturing patent families contain issued patents in the United States, Europe and other territories, as well as numerous pending patent applications.

We have non-exclusively in-licensed from the NIH a patent family relating to the insect cell-based manufacturing of AAV-based vectors. The patents in this family include two issued patents in the United States and one issued patent in Europe, as well as issued patents in other jurisdictions. The standard 20 year term for patents in this family will expire in 2022. This patent family relates to technology used in Glybera and all of our development programs.

We also in-license from the NIH two patent families relating to AAV5-based vectors. These patents are licensed exclusively for AAV5-based therapeutic products to be delivered to the brain or liver for the treatment of human diseases originating in the brain or liver, excluding arthritis-related diseases, and non-exclusively for AAV5-based therapeutic products to treat any human disease in any manner not covered by the exclusive license. The patents in the first family include two issued patents in the United States, one issued patent in Europe and two issued patents in Japan, as well as issued patents and a pending application in other jurisdictions. The standard 20-year term for patents in this family will expire in 2019. This patent family relates to technology used in our AIP, hemophilia B and Sanfilippo B programs. The second family includes one issued U.S. patent with a standard 20-year term that will expire in 2020. This patent family relates to technology used in our Sanfilippo B program. See "Risk Factors—Risks Related to Our Intellectual Property—Our intellectual property licenses with third parties may be subject to disagreements over contract interpretation, which could narrow the scope of our rights to the relevant intellectual property or technology or increase our financial or other obligations to our licensors."

#### *Other Manufacturing Patents*

We own a patent family directed to improved AAV vectors that are stably expressed in insect cells. The family includes an issued patent in the United States and pending applications in the United States, Europe, Japan and other jurisdictions. The standard 20 year term for patents in this family will expire in 2027. This patent family relates to technology used in Glybera and all of our development programs.

We also in-license a patent family related to aspects of the AAV insect cell production technology from Protein Sciences Corporation. This family includes issued patents in the United States, Europe and elsewhere. This license is exclusive in respect of the products we develop with the use of this patent family for LPLD, hemophilia B and AIP, and we may add additional products to the license on an exclusive basis except in certain specified circumstances. The standard 20 year term for patents in this family will expire in 2019. This patent family relates to technology used in Glybera and all of our development programs.

We non-exclusively in-license a family of patents relating to methods for intramuscular administration of AAV vectors from Asklepios Biopharmaceutical, Inc., or AskBio. This family includes issued patents in Europe, Japan and other jurisdictions, and a pending application in the United States. The standard 20 year term for patents in this family will expire in 2016. This patent family relates to technology used in Glybera.

We own a method of manufacturing patent family relating to a second-generation manufacturing method used in our AIP, hemophilia B and Parkinson's disease programs. This patent family contains pending applications in the United States, Europe, Japan and other jurisdictions, and issued patents in several jurisdictions. The standard 20 year term for patents in this family will expire in 2028.

We also own a PCT application that relates to a proprietary baculovirus filtration process. The standard 20 year term for patents in this family, if issued, will expire in 2032. This patent family relates to technology used in Glybera and all of our development programs.

#### *Glybera*

We co-own with University of British Columbia, or UBC, a patent family relating to the lipoprotein lipase variant LPL-S447X transgene used in Glybera, including issued patents in Europe and Japan. The standard 20 year term for patents in this family will expire in 2020. UBC exclusively licensed its patent rights to Xenon, which has granted us the sublicense described below.



We exclusively in-license from Aventis Pharma S.A., or Aventis, a patent family co-owned by UBC and Aventis that relates to the use of AAV-LPL vectors for LPL-deficiency, including issued patents in Europe and other jurisdictions and two pending United States patent applications. The standard 20 year term for patents in this family will expire in 2015.

We own a family of patents relating to a VP1 vector capsid modification, which relates to the production of AAV vectors in insect cells and to AAV vectors with an altered ratio of viral capsid proteins that provides improved infectivity of the viral particles. This patent family includes issued patents in the United States, Europe and elsewhere, as well as pending applications in Europe, Japan and other jurisdictions. The standard 20 year term for patents in this family will expire in 2026.

We non-exclusively in-license a patent family from the Salk Institute that relates to a genetic promoter that enhances the expression of LPL-S447X delivered to the target tissues. This family includes four issued patents in the United States that have standard 20 year terms that will expire in 2017, and issued patents in Europe and other jurisdictions that have standard 20 year terms that will expire in 2018.

We non-exclusively in-license a patent family relating to the AAV1 capsid from AmpliPhi Biosciences, Inc. (formerly Targeted Genetics Corporation), or AmpliPhi. This family includes three issued patents in the United States, and one each in Europe and Japan, as well as issued patents elsewhere and a pending application in the United States. The standard 20 year term for patents in this family will expire in 2019. The University of Pennsylvania exclusively licensed its patent rights to AmpliPhi, which has granted us the sublicense described below.

#### *Other Programs*

**Hemophilia B.** Our patent portfolio covering our hemophilia B program includes an exclusively in-licensed patent family from St. Jude relating to a specific promoter and a codon optimized hFIX transgene. This patent family includes two issued patents in the United States and one in Europe. The United States patent rights will expire in 2028 and the European patents will expire in 2025.

**AIP.** Our patent portfolio covering our AIP program includes a patent family co-owned with Proyecto de Biomedicina Cima S.L. and exclusively licensed to us. This family relates to the codon optimized PBGD transgene and its use for the treatment of AIP. This family includes pending applications in the United States, Europe, Japan and elsewhere. The standard 20 year term for patents in this family will expire in 2029.

**Parkinson's disease.** For our Parkinson's disease program, we have in-licensed a patent family and corresponding know-how relating to the GDNF transgene from Amgen for the field of gene therapy. The license is exclusive and expires on a country-by-country basis on the later of 10 years following launch of the relevant product or of expiration of the last-to-expire licensed patent in the applicable country, after which the license will become non-exclusive for that given country. This patent family includes two issued patents in the United States, one of which will expire in 2015 and one in 2017.

#### **Licenses**

We have obtained exclusive or non-exclusive rights from third parties under a range of patents and other technology that we use in our product and development programs, as described below. Our agreements with these third parties generally grant us a license to make, use, sell, offer to sell and import products covered by the licensed patent rights in exchange for our payment of some combination of an upfront amount, annual fees, royalties, a portion of amounts we receive from our licensees and payments upon the achievement of specified development, regulatory or commercial milestones. Some of the agreements specify the extent of the efforts we must use to develop and commercialize licensed products. The agreements generally expire upon expiration of the last-to-expire valid claim of the licensed patents. Each licensor may terminate the applicable agreement if we materially breach our obligations and fail to cure the breach within a specified cure period, in addition to other termination rights in some cases.

### *Technology Used for Multiple Programs*

We are exploiting technology from the third party sources described below in more than one of our programs.

***National Institutes of Health—AAV production.*** In 2007, we entered into a license agreement with the NIH, which we amended in 2009 and 2013. Under the license agreement, the NIH has granted us a non-exclusive license to patents relating to production of AAV vectors, to make, use, sell, offer to sell and import specified plasmids, which are small DNA molecules that are physically separate from, and can replicate independently of, chromosomal DNA within a cell, or other materials, which we refer to as AAV products. We may only grant sublicenses under this agreement with the NIH's consent, which may not be unreasonably withheld. We are exploiting this technology for our Glybera program and our programs for hemophilia B, AIP, and Sanfilippo B syndrome, and Parkinson's disease.

Payment obligations to the NIH under this license agreement include a one-time upfront payment of \$12,000, which we have paid; a low single-digit percentage royalty on the sale of AAV products by us or on our behalf; a maximum sub-teen double-digit percentage of sublicensing income; potential additional development milestone fees for the initiation of each clinical trial, which would total in the aggregate \$255,000 for one Phase I, Phase II and Phase III trial; potential regulatory milestone fees totaling \$750,000 for the first marketing approvals in specified countries or jurisdictions; and an annual maintenance fee creditable against royalties. We do not have to pay royalties or milestone fees under this agreement if we have to pay royalties or milestone fees under our 2011 agreement with the NIH, described below, for the same product. In connection with entering into our relationship with Chiesi and obtaining the NIH's consent to sublicense our rights under this agreement to Chiesi, we also paid the NIH a total of \$328,684 in amendment and sublicense payments. Under the license agreement, we have agreed to meet benchmarks in our development efforts, including as to development events, clinical trials and marketing approval, within specified timeframes.

The NIH may terminate this agreement in specified circumstances relating to our insolvency or bankruptcy. We may terminate this agreement for any reason, in any territory, subject to a specified notice period.

***National Institutes of Health—AAV5.*** In 2011, we entered into another license agreement with the NIH, which superseded a prior 2007 agreement and which we amended in 2013. Under this agreement, the NIH granted us an exclusive, worldwide license to patents relating to AAV5 for use in therapeutic products to be delivered to the brain or liver for treatment of human diseases originating in the brain or liver, but excluding arthritis-related diseases, and a non-exclusive, worldwide license to patents relating to AAV5 for all other diseases, in each case to make, use, sell, offer to sell and import products within the scope of the specified patent claims. We refer to the products licensed under this agreement as AAV5 products. We may grant sublicenses under this agreement only with the NIH's consent, which may not be unreasonably withheld. We are currently exploiting this technology for our programs on hemophilia B, AIP, and Sanfilippo B syndrome. See "Risk Factors—Our intellectual property licenses with third parties may be subject to disagreements over contract interpretation, which could narrow the scope of our rights to the relevant intellectual property or technology or increase our financial or other obligations to our licensors."

We have agreed to pay the NIH an initial payment of \$140,000, which we have paid, an amendment royalty fee of \$500,000, of which \$250,000 would be payable upon a sublicense of the corresponding rights, which we have paid in full, royalties equal to a low single-digit percentage of net sales of AAV5 products, if any, by or on behalf of us or our sublicensees; a single to subteen double-digit percentage of sublicensing income; potential additional development milestone fees for the initiation of each clinical trial, which would total in the aggregate \$267,500 for one Phase I, Phase II and Phase III trial; total potential regulatory milestone fees of \$1,731,000 for the first marketing approvals in specified countries or jurisdictions; and an annual maintenance fee creditable against royalties. In connection with entering into our relationship with Chiesi and obtaining the NIH's consent to sublicense our rights under this agreement to Chiesi, we paid the NIH a total of \$716,567 in amendment and sublicense payments. If an AAV5 product is also

covered by our 2007 agreement with the NIH, our obligation to pay royalties on net sales and our obligation to pay milestone fees only apply under this 2011 agreement and not the 2007 agreement. We have agreed to meet benchmarks in our development efforts, including as to development events, clinical trials and marketing approval, within specified timeframes.

The NIH may terminate this agreement in specified circumstances relating to our insolvency or bankruptcy. We may terminate this agreement for any reason, in any country or territory, subject to a specified notice period.

**Protein Sciences.** In 2007, we entered into a license agreement with Protein Sciences Corporation, or PSC, which we amended in 2012. Under the license agreement, PSC granted us a worldwide license, with a right to sublicense, to specified claims of a patent relating to an insect cell line, to research, develop, manufacture, import, market, and to offer for sale and sell certain products using a recombinant AAV vector developed using PSC's technology. The license is exclusive with respect to LPLD, hemophilia B and AIP, and we are exploiting this technology for those programs. We are licensed to use this technology for products listed in the agreement and we may add additional products to the agreement on an exclusive basis except in certain specified circumstances.

Payments obligations under the PSC agreement include a one-time upfront payment of \$50,000, which we have paid, payments of \$50,000 for each additional product added to the license agreement, and an annual maintenance fee for each product up to an annual maximum and limited by an overall specified life-time maximum dollar amount for each product. We are not required to pay maintenance fees on products we no longer wish to develop. In addition, we must pay PSC an annual fee while any product is being sold or is subject to a license, partnership or funding relationship with another party, but for no more than 10 years after the first commercial sale of the product. We have no royalty payment obligations under the agreement.

The agreement will remain in effect as long as we remain current with our payments or until we or PSC exercise our rights to terminate it. PSC may terminate the agreement in circumstances relating to our insolvency or bankruptcy. We may terminate the agreement for convenience subject to a specified notice period.

#### *Technology Used for Specific Programs*

##### *Glybera*

We are exploiting technology from the following third party sources in our Glybera program.

**Academic Medical Center at the University of Amsterdam.** In 2006, we entered into an agreement with the Academic Medical Center at the University of Amsterdam, or AMC, and certain other parties, through which AMC invested in our predecessor company. Under this agreement, AMC assigned patent rights to us relating to LPLD and certain other indications.

We have agreed to pay AMC royalties equal to a low single-digit percentage of net sales, if any, of gene therapies to treat LPLD or certain other indications sold by us or our sublicensees that are covered by the assigned patent.

We have agreed to use commercially reasonable and diligent efforts to obtain marketing approvals for, and to commercialize, gene therapies to treat LPLD and certain other indications. If we decide to cease developing and commercializing a product to treat LPLD or certain other indications in each of Europe, the United States and Canada, we must re-assign to AMC the patent rights related to that product upon AMC's request.

**Xenon Genetics, Inc.** In 2001, we entered into a sublicense agreement with Xenon Genetics, Inc., or Xenon, which we subsequently amended. Under the sublicense agreement, Xenon has granted us an exclusive, worldwide sublicense to patents and related technology relating to a truncated form of the LPL

protein, to use, manufacture, distribute and sell products using the licensed patents or technology. We may only grant sublicenses under this agreement with consent of Xenon and its licensor UBC.

Payment obligations under the agreement include an initial sublicense fee of Canadian dollars C\$75,000 and a one-time upfront payment to Xenon in the total amount of C\$600,000, both of which we have paid, payment of certain past and future patent costs, a mid-single-digit percentage royalty on net sales, if any, of licensed products sold by us or our affiliates while covered by a valid patent claim, a low single-digit percentage royalty in countries where no patent protection covers the products, a double digit percent share of the royalties paid to us by Chiesi and an equal or slightly higher share of royalties paid to us by other sublicensees in other specified circumstances. The share of the royalty we receive from Chiesi and any other sublicensee that we have agreed to pay to Xenon decreases to a mid-single digit percentage after patent coverage expires, and the obligation terminates 10 years after the first commercial sale of the product. We have also agreed to pay Xenon development milestone fees totaling a maximum of C\$350,000, plus an additional maximum of C\$200,000 per additional product for a different indication upon our achievement of specified development milestones, as well as fees upon our achievement of specified regulatory milestones totaling a maximum of C\$400,000 plus an additional maximum of C\$400,000 per additional product for a different indication; or, if higher, a double digit percent share of any non-royalty fees we receive from a sublicensee.

The agreement will remain in effect until we or Xenon exercise our rights to terminate it. Either party may terminate the agreement in circumstances relating to the other party's insolvency or bankruptcy.

**Aventis.** In 2006, we entered into a license agreement with Aventis Pharma, S.A., or Aventis, which we amended in 2013. Under the license agreement, Aventis has granted us an exclusive license, with a right to sublicense, to patents owned by Aventis and co-owned by Aventis and UBC, to develop, use, make, sell and offer to sell gene therapies containing a recombinant virus with an LPL gene.

Under the agreement, we made a one-time upfront payment to Aventis of €10,000 and agreed to pay Aventis a high single-digit to sub-teen double-digit royalty as a percentage of our net sales of licensed products, or if sales are made by a commercialization partner, a low single-digit as a percentage of net sales royalty, or, if higher, a high single-digit to sub-teen double-digit royalty as a percentage of royalties we receive from such commercialization partner plus an equivalent percentage of the price we invoice the commercialization partner for the licensed products less our cost of goods sold, subject to a floor of a low single-digit percentage of net sales by Chiesi or another commercialization partner. We have also agreed to pay Aventis a one-time milestone fee of €50,000 upon our achievement of a specified regulatory milestone and €75,000 upon our achievement of a specified commercial milestone.

In conjunction with amending the agreement in 2013, we have agreed to provide Aventis with a right of first negotiation regarding a specified product candidate to treat AIP if, at the time we complete Phase I/II clinical trials of the product candidate or within a specified period thereafter, we contemplate entering into a partnership for the co-development and commercialization of the product candidate.

The agreement will remain in effect until the expiration of the protection provided by the licensed patents, or until we or Aventis exercise our rights to terminate it. Aventis may terminate the agreement in circumstances relating to our bankruptcy.

**Asklêpios Biopharmaceutical.** In 2010, we entered into a license agreement with AskBio under which AskBio granted us a non-exclusive, worldwide license, with a right to sublicense, to patents relating to administration of an AAV vector to muscle tissue for use in treatment of LPLD with Glybera or other products that contain an AAV vector having an AAV genetic construct encoding an LPL gene variant, to research, develop, make, use, sell, offer for sale, and import the products to treat LPLD.

We made a one-time upfront payment to AskBio of \$50,000 and have agreed to pay AskBio annual maintenance fees during the term of the license.

The agreement will remain in effect on a country-by-country basis until the earlier of June 5, 2016 or the expiration of the last to expire of the valid claims in the licensed patents. We may terminate the agreement for convenience at any time subject to a specified notice period.

***Salk Institute for Biological Studies.*** In 2008, we entered into a license agreement with the Salk Institute for Biological Studies, or Salk, which we amended in 2013. Under the license agreement, Salk has granted us a non-exclusive license to specified biological materials and patents relating to a DNA promoter, to research, develop, make, use, import, offer for sale, and sell products using their technology for gene therapy. We have a right to enter into sublicenses under this agreement, subject to prior written consent by Salk, which may not be unreasonably withheld, and to other conditions.

Payment obligations under the agreements include an upfront payment of \$35,000 in 2008 and \$5,000 in 2013 in connection with an amendment and consent to sublicense to Chiesi, both of which we have paid, as well as annual maintenance fees, a royalty equal to a low single-digit percentage of net sales, if any, of licensed products sold by us, or, if higher, by Chiesi, and payments of a low single-digit percentage of all execution fees, maintenance fees, milestone fees and other non-royalty payments received by us from Chiesi or any other sublicensee.

The agreement will remain in effect on a country-by-country basis until the latest of 15 years from the effective date, the date of expiration of the last to expire licensed patent or the abandonment of the last remaining licensed patent application.

***AmpliPhi Biosciences.*** In 2006, we entered into a license agreement with AmpliPhi (formerly Targeted Genetics Corporation), which we amended in 2013. Under the license agreement, AmpliPhi has granted us a non-exclusive, worldwide sublicense to patents exclusively licensed by AmpliPhi from the University of Pennsylvania, or Penn, relating to AAV1, to make, develop, use, sell, offer to sell and import products using the patent rights to treat LPLD type 1, which includes the Glybera patient population, and LPLD type 5 by in vivo gene therapy. We may only grant sublicenses under this agreement with the consent of AmpliPhi and Penn, which may not be unreasonably withheld.

We have to date paid to AmpliPhi a one-time up-front payment of \$1,750,000. We have agreed to pay AmpliPhi annual fees, a total of \$4,950,000 in development and regulatory milestone payments, which we have paid, and a royalty equal to a low single-digit percentage of net sales, if any, of licensed products sold by us or Chiesi.

Either party may terminate the agreement in circumstances relating to the other party's insolvency or bankruptcy. We may terminate the agreement for convenience at any time subject to a specified notice period.

If the agreement is terminated by us due to AmpliPhi's insolvency, bankruptcy or material uncured breach, or if AmpliPhi's license agreement with Penn is terminated, our license from AmpliPhi may be assigned to Penn. The assignment must be made on our request but is at Penn's discretion, which Penn may not unreasonably withhold, provided that the agreement specifies that Penn's obligations are consistent with its current obligations and provided that we assume all AmpliPhi's obligations.

#### *Hemophilia B*

***St. Jude Children's Research Hospital.*** In 2008, we entered into a license agreement with St. Jude, which we amended in 2012. Under the license agreement, St. Jude has granted us an exclusive license, with a right to sublicense, to patent rights relating to expression of hFIX in gene therapy vectors, to make, import, distribute, use and commercialize products containing hFIX covered by a valid patent claim in the field of gene therapy for treatment or prophylaxis of hemophilia B. In addition, we have a first right of negotiation regarding any patent applications that are filed by St. Jude for any improvements to the patent rights licensed to us.

We have agreed to pay St. Jude a royalty equal to a low single-digit percentage of net sales, if any, by us or our sublicensees of products covered by the licensed patent rights, and a portion of certain amounts we receive from sublicensees ranging from a mid-single digit to a mid-teen double digit percentage of such amounts. We have also agreed to pay St. Jude one-time milestone fees totaling \$6,500,000 upon the achievement of specified development and regulatory milestones, and an annual maintenance fee creditable against royalties and milestones in the same year. We have agreed to use commercially reasonable efforts to diligently develop and commercialize products licensed under this agreement.

The agreement will remain in effect until no further payment is due relating to any licensed product under this agreement or either we or St. Jude exercise our rights to terminate it. St. Jude may terminate the agreement in specified circumstances relating to our insolvency. We may terminate the agreement for convenience at any time subject to a specified notice period.

#### *AIP*

**Digna Biotech.** In 2010, we entered into a license agreement with Digna Biotech, S.L, or Digna Biotech, Fundación para la Investigación Médica Aplicada, or FIMA, the members of a collaborative research consortium known as UTE CIMA, and Proyecto de Biomedicina CIMA S.L., or Proyecto, which superseded several prior agreements amongst such parties. We refer to Digna Biotech, FIMA, UTE CIMA and Proyecto collectively as the CIMA Parties. Under the license agreement, Proyecto granted us an exclusive, worldwide license, with a right to sublicense, under its interest in patent rights we jointly own with Proyecto relating to PBGD gene therapy to use, develop, make, have made and commercialize products using the licensed patent rights. In addition, UTE CIMA granted us a non-exclusive, worldwide license, with the right to grant sublicenses, under certain patent rights, know-how and materials required for the use, development, manufacture or commercialization of products covered by our exclusive license from Proyecto in the gene therapy field.

We have agreed to pay Digna Biotech royalties equal to a mid-single digit percentage of net sales, if any, by us or our affiliates of licensed products covered by our exclusive license and a sub-teen double-digit percentage share of net revenues we receive from our sublicensees. Digna Biotech is responsible for apportioning the amounts we pay Digna Biotech amongst the CIMA Parties.

Under the agreement we have to use commercially reasonable efforts to further develop, manufacture and commercialize licensed products as soon as reasonably practicable.

The agreement will remain in effect until our payment obligations expire or we or another party exercise our rights to terminate it. A party may terminate the agreement in circumstances relating to another party's insolvency or bankruptcy or if our agreement under which Digna Biotech is conducting a Phase I clinical trial of AMT-021 terminates. We may terminate this agreement for convenience, subject to a specified notice period. If Digna Biotech terminates the license agreement for breach or insolvency, we or Digna Biotech terminate the license agreement because our other agreement with Digna Biotech terminates other than for breach or insolvency of Digna Biotech or if we terminate the license agreement for convenience, the CIMA Parties will have the exclusive right to use the patent rights we jointly own with Proyecto that were exclusively licensed to us to further develop and commercialize licensed products for the treatment or prevention of AIP without financial obligations to us.

#### *Parkinson's disease*

**Amgen.** In 2010, we entered into a license agreement with Amgen, Inc. which superseded a prior 2008 agreement. Under the license agreement, Amgen granted us an exclusive, worldwide license, with a right to sublicense, to patents and know-how relating to GDNF to research, develop, make, use, offer for sale, sell, import, export and otherwise exploit gene therapies capable of delivering GDNF, the gene encoding GDNF, or any fragment of GDNF that has specified functional activity, which we refer to as GDNF products. The license exclusivity, and our obligation to make the revenue sharing payments described below, with respect

to a given GDNF product in a given country expires on the later of expiration of the last-to-expire licensed patent in such country that covers such GDNF product and the tenth anniversary of the first commercial sale of such GDNF product in such country. Thereafter the license would become non-exclusive with respect to that GDNF product in that country.

We have agreed to pay Amgen revenue sharing payments equal to a sub-teen double-digit percentage of net revenues, if any, that we receive from our sales of GDNF products, from granting sublicenses under the intellectual property licensed from Amgen or from granting licenses under certain of our intellectual property rights. Upon receipt of the first marketing approval anywhere in the world for the first GDNF product we have also agreed to pay Amgen a one-time milestone fee of the greater of \$10 million or a sub-teen double digit percentage of any milestone payments we receive from third parties with respect to receiving such approval.

We agreed to use reasonably diligent efforts to develop at least one GDNF product and seek to obtain regulatory approvals for this GDNF product in the United States and the European Union, and to commercialize it.

We granted Amgen an option to negotiate an exclusive license from us to research, develop, make, use, offer for sale, sell and otherwise exploit GDNF products in the United States, Mexico and Canada. Amgen may exercise the option within a specified period following completion of the first Phase II clinical trial of the first GDNF product we develop. If Amgen exercises the option but we and Amgen do not execute a definitive agreement to grant these rights to Amgen within a specified period of time, we retain these rights but may not grant development or commercialization rights to a third party in these North American countries on financial terms less favorable to us than those last offered by Amgen.

The agreement will remain in effect until either we or Amgen exercise our rights to terminate it. We may terminate the agreement for convenience at any time subject to a specified notice period. If we terminate the agreement for convenience, or if Amgen terminates the agreement due to our uncured material breach, rights to GDNF products will revert to Amgen. As part of such reversion, if Amgen requests, we have agreed to grant Amgen an exclusive, worldwide license under our relevant intellectual property rights so that Amgen can research, develop, make, use, offer for sale, sell and otherwise exploit GDNF products, subject to a specified revenue sharing and a one-time regulatory milestone payment from Amgen to us.

**UCSF.** In 2012, we entered into a data license agreement with the University of California in San Francisco, or UCSF, related to UCSF's rights to the clinical trial data from a Phase I/II clinical trial, sponsored by the NIH, and that UCSF is conducting, of a product candidate consisting of an AAV2 vector carrying the GDNF gene, and to certain related preclinical data and know-how. Under the data license agreement, UCSF granted us a non-exclusive license, with a right to sublicense, to research, develop, make, use, offer for sale, sell and otherwise exploit pharmaceutical products containing or consisting of an AAV2 genetic construct encoding GDNF, or any fragment of GDNF that has specified functional activity, for the therapeutic, palliative and prophylactic treatment of Parkinson's disease in humans. During the term of the data license agreement, UCSF has agreed not to grant to any other for-profit entity any of the rights granted to us thereunder, except under specified circumstances involving a breach of our diligence obligations described below.

Payment obligations under the agreement include a one-time, up-front payment of \$300,000, which we have paid, a royalty equal to a low single-digit percentage of our net sales, if any, of products that are identified or developed through material use of the data licensed from UCSF, or identified products, as well as third party license fees with the percentage due to UCSF ranging from a low double-digit percentage for earlier-granted sublicenses to a low single-digit percentage for later-granted sublicenses. Our obligation to pay UCSF earned royalties with respect to a given country begins on the first commercial sale of an identified product in such country, and our obligation to pay earned royalties and third-party license fees



expires on the tenth anniversary of such first commercial sale, after which the data license will become perpetual, non-exclusive, fully paid-up, and royalty-free in such country.

The UCSF agreement also contains certain other obligations we have agreed to complete by specified dates, including obligations to deliver to UCSF by June 12, 2014 specified materials for UCSF to complete a non-clinical study of an AAV2 vector carrying the GDNF gene, to demonstrate equivalent product release specifications of our vector to the vector used in the ongoing NIH-sponsored Phase I clinical trial, to pursue a bridging study using our AAV2 vector carrying a GDNF gene, and to use commercially reasonable efforts to proceed, either directly or through a third party licensee, to develop, seek to obtain regulatory approval for and market at least one identified product in the United States and the European Union.

If we materially fail to comply with any of the diligence obligations described above and do not cure such failure within specified cure periods, UCSF may at its option either terminate the data license agreement or be freed from its covenant not to grant to any other for-profit entity any of the rights granted to us thereunder.

The data license agreement will remain in effect until all of our payment obligations to UCSF have ended in all countries, unless either we or UCSF exercise our rights to terminate it earlier. UCSF may terminate the agreement in specified circumstances relating to our bankruptcy. We may terminate the agreement for convenience at any time subject to a specified notice period.

### **Trade Secrets**

In addition to patents and licenses, we rely on trade secrets and know-how to develop and maintain our competitive position. For example, significant aspects of the process by which we manufacture Glybera and our gene therapies are based on unpatented trade secrets and know-how. We seek to protect our proprietary technology and processes and obtain and maintain ownership of certain technologies, in part, through confidentiality agreements and invention assignment agreements with our employees, consultants, scientific advisors, contractors and commercial partners. We also seek to preserve the integrity and confidentiality of our data, trade secrets and know-how by maintaining physical security of our premises and physical and electronic security of our information technology systems.

### **Trademarks**

uniQure and Glybera are registered trademarks in various jurisdictions including the United States and the European Union. We intend to seek trade mark protection for other product candidates as and when appropriate.

### **Strategic Collaboration**

In April 2013, we entered into two agreements with Chiesi. One is an agreement for the commercialization of Glybera for LPLD and the second is an agreement for the co-development and commercialization of our hemophilia B program. To date, under these two collaborations we have received €17.0 million in upfront payments aggregate non-equity funding, as well as a €14.0 million investment in our ordinary shares. Additionally, the agreements provide us with research funding for further development of our hemophilia B product candidate, the potential for commercial milestone payments of up to €42.0 million for Glybera for LPLD, and payments for commercial quantities of Glybera we supply to Chiesi. We summarize the key terms of these two agreements below.

#### **Glybera for LPLD**

**Overview.** In April 2013, we entered into an agreement with Chiesi to commercialize Glybera for LPLD in the following countries, which we refer to as the Chiesi Glybera territory:

- the then current 27 member states of the European Union plus Iceland, Liechtenstein and Norway;



- Albania, Andorra, Bosnia, Croatia, Macedonia, Monaco, Montenegro, Republic of San Marino, Serbia (including Kosovo), Switzerland and Vatican City; and
- Algeria, Brazil, China, Egypt, Mexico, Morocco, Pakistan, Russia and ex-Soviet countries (Armenia, Azerbaijan, Belarus, Georgia, Kazakhstan, Kirghizstan, Moldova, Tajikistan, Turkmenistan, Ukraine and Uzbekistan), Tunisia and Turkey.

In all other countries of the world, including the United States and Japan, we retain all product rights to Glybera for LPLD.

Under the terms of the agreement, we received a €2.0 million upfront payment in recognition of our past expenditures developing the product. In addition, we are eligible to earn up to €42.0 million in commercial milestone payments. We will receive payment for individual quantities of the product we manufacture and supply under the agreement, payable partly upon order and partly following delivery of such product quantities, and amounting to the greater of (1) 40% of the average net sales price of the product and (2) the fully loaded cost of goods plus 20% mark-up for each patient dose sold.

Under the agreement, we appointed Chiesi as our distributor with the exclusive right to commercialize, itself or through affiliates, with our written consent, or other sub-distributors, Glybera for LPLD in the Chiesi Glybera territory. The appointment includes an exclusive license grant to our trademark "Glybera" in the Chiesi Glybera territory for the purposes of the agreement, as well as a license to certain intellectual property rights controlled by us. Chiesi is generally required to commercialize the product exclusively under the "Glybera" name.

Under the terms of the agreement, Chiesi does not have the right to carry out any research or development with respect to Glybera or to manufacture Glybera or have Glybera manufactured, except for certain exceptions, such as our failure to supply the product to them. During the term of the agreement, we are required to manufacture and supply, and Chiesi is required to purchase from us all of its requirements for Glybera for commercialization under the agreement.

*Commercialization Obligations.* Chiesi has the sole right and responsibility to commercialize Glybera in the Chiesi Glybera territory at its expense using no less than commercially reasonable efforts, including compliance with an agreed marketing plan and budget and the allocation of an agreed minimum workforce to the commercialization of the product. Chiesi will also be responsible for conducting price and reimbursement submissions to the relevant reimbursement bodies. We and Chiesi have allocated between us responsibilities for the filing, holding and maintenance associated with marketing authorizations for Glybera in the various countries and jurisdictions covered by our agreement, as well as associated costs.

*Governance.* Our collaboration with Chiesi is guided by a joint steering committee and a joint commercialization committee. Subject to limitations specified in the agreement, if the applicable governance committee is not able to make a decision by consensus and the parties are not able to resolve the issue through escalation to the superior committee or, in some cases, specified senior executive officers of the parties, then:

- we have final decision-making authority with respect to all matters related to research or development in relation to Glybera, with reasonable input from Chiesi taking into account territory-specific matters;
- Chiesi has final decision-making authority with respect to all matters related to commercialization of Glybera in the Chiesi Glybera territory, with reasonable input from us taking into account our global product strategy;
- on regulatory matters with respect to Glybera we generally will jointly work with Chiesi towards a regulatory strategy for Glybera in the countries of the Chiesi Glybera territory that are not member states of the European Union; and
- any other matter will be decided by binding arbitration.

**Exclusivity Restrictions.** During the term of the agreement, we may not offer for sale, sell, license or otherwise commercialize Glybera in the Chiesi Glybera territory other than in compliance with the terms of the agreement. Moreover, to the fullest extent consistent with applicable laws, each of Chiesi and we may not, directly or indirectly, develop, manufacture or commercialize in the Chiesi Glybera territory any gene therapy-based product for the treatment of LPLD, other than Glybera in accordance with the terms of the agreement.

**Term and Termination.** Our agreement with Chiesi will remain in force, on a country-by-country basis, until the latest of:

- 12 years from the first commercial sale of Glybera in the relevant country;
- expiry of any regulatory exclusivity granted by any marketing authorization or any other regulatory approval in the relevant country; or
- expiry of the last valid claim of specified patent rights covering Glybera in the relevant country.

Unless terminated by a party with three months written notice to the other party prior to the end of the initial or any subsequent term, the agreement will automatically renew for successive five year terms. Chiesi has the right to terminate the agreement in its entirety in case of our failure to supply Glybera for a period of at least nine months, provided such failure to supply is not caused by a force majeure event and other pre-conditions for termination are met. Either party may also terminate the agreement in its entirety in the event of a material breach by the other party, in the event the other party becomes subject to specified bankruptcy, insolvency or similar circumstances, or if the other party or any of its affiliates or, as the case may be, sub-distributors challenges the validity of any trademark to which rights are granted under the agreement. We may terminate the agreement in its entirety if Chiesi or any of its affiliates or sub-distributors challenges the validity, enforceability, patentability or scope of any valid claim included in any of the patents covering Glybera. We may also terminate this agreement in its entirety or with respect to particular countries if Chiesi fails to meet certain commercialization requirements and such failure is not caused by a force majeure event or our failure to supply. After termination in certain circumstances, we will have continuing supply obligations.

### **Hemophilia B (AMT-060)**

**Overview.** In April 2013, we entered into a co-development and license agreement with Chiesi in respect of our hemophilia B gene therapy program in the following countries, which we refer to as the Chiesi hemophilia B territory:

- the then current 27 member states of the European Union plus Iceland, Liechtenstein and Norway; and
- Albania, Algeria, Andorra, Bosnia, Brazil, Croatia, Egypt, Macedonia, Mexico, Monaco, Montenegro, Morocco, Pakistan, Republic of San Marino, Russia and ex-Soviet countries (Armenia, Azerbaijan, Belarus, Georgia, Kazakhstan, Kyrgyzstan, Moldova, Tajikistan, Turkmenistan, Ukraine and Uzbekistan), Serbia (including Kosovo), Switzerland, Tunisia, Turkey, and Vatican City.

In all other countries of the world, including the United States, China and Japan, we retain all product rights to our hemophilia B program.

Under the terms of the agreement, we received a €15.0 million upfront payment. In addition, we will share equally with Chiesi specified development costs attributable to the program according to a defined development plan and budget, including costs associated with preclinical and clinical studies as well as development and regulatory milestone payments associated with existing in-license agreements. We will receive payments from Chiesi for commercial quantities of our hemophilia B product candidate we manufacture and supply to them, if we receive regulatory approval for such product candidate. We estimate that the amount we would retain, net of cost of goods sold, including third party royalties and related amounts, will be between 25% and 35% of the revenues from sales of such product by Chiesi, varying by

country of sale. We and Chiesi have agreed to negotiate a separate supply and distribution agreement in respect of the potential commercialization of our hemophilia B product candidate prior to dosing the first patient in any pivotal study. We are not entitled to any milestone payments under this agreement.

Under the agreement, we granted to Chiesi and its affiliates an exclusive license, for the Chiesi hemophilia B territory, to co-develop, together with us, and exclusively commercialize a gene therapy for the treatment of hemophilia B in humans that includes an AAV5 vector containing a functional copy of the codon-optimized hFIX gene or part thereof under the control of a liver-specific promoter. Chiesi granted to us the right to use relevant data related to such product and generated under this development program in connection with development and regulatory activities conducted by us or on our behalf outside of the Chiesi hemophilia B territory.

*Research Obligations.* We and Chiesi must use commercially reasonable efforts to conduct the activities assigned to each of us under the agreed development plan and budget. Specifically, we are responsible for all activities to develop the product in the Chiesi hemophilia B territory, including all clinical development activities required to obtain marketing authorization in the Chiesi hemophilia B territory, with particular emphasis on France, Germany, Italy, Spain and the United Kingdom, and must provide supplies of the product as necessary for the activities under the development plan and budget. We are also obligated to provide commercial supply of the product to Chiesi pursuant to a supply and distribution agreement, which we shall negotiate as noted above. Chiesi is responsible for all launch and pre-launch activities for the product in the territory, including regulatory filings and approvals, interaction with regulatory authorities, key opinion leader development, market research and pricing and reimbursement studies.

*Governance.* Our collaboration with Chiesi is initially guided by at least a joint steering committee and a joint development committee. Subject to limitations specified in the agreement, if the applicable governance committee is not able to make a decision by consensus and the parties are not able to resolve the issue through escalation to the superior committee or, in some cases, specified senior executive officers of the parties, then:

- we will generally have final decision-making authority with respect to all research and development activities with respect to the product, with reasonable input from Chiesi taking into account territory-specific matters;
- Chiesi will generally have final decision-making authority with respect to all commercialization activities with respect to the product in the territory, with reasonable input from us taking into account our global product strategy; and
- certain items specifically identified in the agreement will require a unanimous decision of both parties.

With respect to regulatory matters, we and Chiesi will jointly work towards a regulatory strategy for the licensed product in the Chiesi hemophilia B territory, with an understanding that Chiesi will have the final decision right on the regulatory strategy for the product in the territory, and will support our global regulatory strategy for the licensed product unless such support leads to a material increase in costs or time to market for Chiesi. Any other matter will be decided by binding arbitration.

*Exclusivity Restrictions.* During the term of the agreement neither party may, directly or indirectly, undertake the development, manufacture or commercialization anywhere in the Chiesi hemophilia B territory of any gene therapy for hemophilia B in humans other than the licensed product.

*Term and Termination.* Our agreement with Chiesi will remain in force, on a country-by-country basis, until the latest of:

- 12 years from the first commercial sale of the licensed product in the relevant country;
- expiry of any regulatory exclusivity granted by any marketing authorization or any other regulatory approval in the relevant country; or
- expiry of the last valid claim of the licensed patents covering the licensed product in the relevant country.

Unless terminated by a party with three months written notice prior to the end of the initial or any subsequent term, the agreement will automatically renew for successive five-year terms. Chiesi may terminate the agreement for convenience upon six months' prior notice to us at any time during the term following the first six months of the agreement. Chiesi also has the right to terminate the agreement in its entirety in case of our failure to supply the licensed product for a period of at least nine months, provided such failure to supply is not caused by a force majeure event. The agreement may also be terminated in its entirety by either party in the event of a material breach by the other party, or if the other party or any of its affiliates or third party contractors challenges the validity, enforceability, patentability or scope of any claim included in any licensed patent.

## Competition

The biotechnology and pharmaceutical industries, including in the gene therapy field, are characterized by rapidly advancing technologies, intense competition and a strong emphasis on intellectual property. We face substantial competition from many different sources, including large and specialty pharmaceutical and biotechnology companies, academic research institutions and governmental agencies and public and private research institutions.

We are aware of several companies focused on developing gene therapies in various indications, including bluebird bio, Sangamo BioScience, AGTC, Oxford Biosciences, Spark Therapeutics, Audentes Therapeutics, RegenX and Asklepios, as well as several companies addressing other methods for modifying genes and regulating gene expression. Although companies and research institutions in the gene therapy field tend to focus on particular target indications, any advances in gene therapy technology made by a competitor may be used to develop therapies competing against Glybera or one of our product candidates. We may also face competition with respect to the treatment of some of the diseases that we are seeking to target with our gene therapies from protein pharmaceuticals under development at pharmaceutical and biotechnology companies such as Pfizer, Baxter, Bayer, Novo Nordisk, Genzyme, Shire, BioMarin, Biogen Idec and numerous other pharmaceutical and biotechnology firms.

We must also compete with existing standards of care, therapies and symptomatic treatments, as well as any new therapies that may become available in the future for the indications we are targeting. For example, for our internal programs, our competition includes the following:

- **Glybera.** The current standard of care for the treatment of LPLD is a restricted diet. We are aware that Novartis is developing a diacylglycerol acyltransferase-1, or DGAT-1, inhibitor, which is currently in a Phase III clinical trial for the treatment of familial chylomicronemia syndrome, and which could compete with Glybera as a treatment alternative for LPLD for a subset of patients if proven effective and approved for marketing.
- **Hemophilia B.** Hemophilia B is typically treated through a program of protein replacement therapy. There are a number of companies that manufacture and market protein therapies for this condition. Biogen Idec has filed a BLA with the FDA seeking approval to market and distribute a longer-acting protein replacement therapy product that would only have to be administered twice a month as opposed to several times a week. In addition, we understand that several companies are developing gene therapies for hemophilia B. Asklepios and Spark Therapeutics have announced Phase I/II studies with AAV-based gene therapy approaches.

Many of our current or potential competitors, either alone or with their collaboration partners, have significantly greater financial resources and expertise in research and development, manufacturing, preclinical testing, conducting clinical trials, and marketing approved products than we do. Mergers and acquisitions in the pharmaceutical, biotechnology and gene therapy industries may result in even more resources being concentrated among a smaller number of our competitors. Smaller or early-stage companies may also prove to be significant competitors, particularly through collaborative arrangements with large and established companies. These competitors also compete with us in recruiting and retaining qualified

scientific and management personnel and establishing clinical trial sites and patient registration for clinical trials, as well as in acquiring technologies complementary to, or necessary for, our programs.

Our commercial opportunity could be reduced or eliminated if our competitors develop and commercialize products that are safer, more effective, have fewer or less severe side effects, are more convenient or are less expensive than any products that we may develop. Our competitors also may obtain FDA or other regulatory approval for their products more rapidly than we may obtain approval for ours, which could result in our competitors establishing a strong market position before we are able to enter the market. The key competitive factors affecting the success of all of our programs are likely to be their efficacy, safety, convenience, price and the availability of reimbursement from government and other third party payors. We also believe that, due to the small size of the patient populations in the orphan indications we target, being first to market will be a significant competitive advantage. We believe that our advantages in vector and manufacturing technology will allow us to reach market in a number of indications ahead of our competitors, and to capture the markets in these indications.

We believe that our EMA-approved, commercially scalable, economically feasible manufacturing process provides a significant competitive advantage in the gene therapy field. We also believe that, in having addressed the manufacturing challenges historically associated with this field, we have positioned ourselves as a key collaboration partner for academic research institutions with exciting early stage pre-clinical programs. We expect that this will help to keep us in the forefront of the field in the development of gene therapies for rare and other diseases.

## **Government Regulation and Reimbursement**

Government authorities in the United States, European Union and other countries extensively regulate, among other things, the approval, research, development, pre-clinical and clinical testing, manufacture (including any manufacturing changes), packaging, storage, recordkeeping, labeling, advertising, promotion, distribution, marketing, post-approval monitoring and reporting, and import and export of pharmaceutical products, biological products and medical devices. We believe that all of our product candidates will be regulated as biological products, or biologics, and in particular, as gene therapies, and will be subject to such requirements and regulations under U.S. and foreign laws.

### ***Regulation in the United States***

In the United States, the Food and Drug Administration, or FDA, regulates biologics under the Public Health Service Act, or PHSA, and the Federal Food, Drug, and Cosmetic Act, or FDCA, and regulations and guidances implementing these laws. Obtaining regulatory approvals and ensuring compliance with applicable statutes and regulatory requirements entails the expenditure of substantial time and financial resources. The failure to comply with applicable requirements may subject an applicant and/or sponsor to a variety of administrative or judicial sanctions, including refusal by FDA to approve pending applications, withdrawal of a license, imposition of a clinical hold, issuance of warning letters and other types of letters, product recalls, fines, and civil or criminal investigations and penalties brought by the Department of Justice and other federal and state government agencies.

All of our current product candidates are subject to regulation by the FDA as biologics. An applicant seeking approval to market and distribute a new biologic in the United States must typically undertake the following:

- completion of preclinical laboratory tests, animal studies and formulation studies in compliance with FDA's good laboratory practice, or GLP, regulations;
- submission to FDA of an Investigational New Drug, or IND Application, which allows human clinical trials to begin unless the FDA objects within 30 days;

- approval by an independent institutional review board, or IRB, representing each clinical site before each clinical trial may be initiated;
- performance of adequate and well-controlled human clinical trials in accordance with FDA's or EMA's good clinical practices, or GCP, to establish the safety, potency, purity and efficacy of the proposed biological product for each indication;
- preparation and submission to FDA of a Biologics License Application, or BLA;
- satisfactory review of the BLA by an FDA advisory committee, when appropriate or if applicable;
- satisfactory completion of one or more FDA inspections of the manufacturing facility or facilities at which the product, or components thereof, are produced to assess compliance with current Good Manufacturing Practices, or cGMP, requirements and to assure that the facilities, methods and controls are adequate to preserve the product's identity, strength, quality and purity;
- payment of user fees and securing FDA approval of the BLA; and
- compliance with any post-approval requirements, including Risk Evaluation and Mitigation Strategies, or REMS, and post-approval studies required by FDA.

### ***Human Clinical Studies Under an IND***

Clinical trials involve the administration of the investigational biologic to human subjects under the supervision of qualified investigators in accordance with GCP requirements. A protocol for each clinical trial and any subsequent protocol amendments must be submitted to FDA as part of an IND. A clinical trial may not proceed unless and until an IND becomes effective, which is 30 days after its receipt by FDA unless before that time FDA raises concerns or questions related to one or more proposed clinical trials and places the trial on clinical hold.

In addition, an IRB at each institution participating in the clinical trial must review and approve the plan for any clinical trial before it commences at that institution, and the IRB must conduct continuing review and reapprove the study at least annually. An IRB must operate in compliance with FDA regulations, and information about certain clinical trials must be submitted within specific timeframes to the NIH for public dissemination on their ClinicalTrials.gov website.

Human clinical trials are typically conducted in three sequential phases, which may overlap or be combined:

- Phase I: The biological product is initially introduced into healthy human subjects or patients with the target disease or condition and tested for safety, dosage tolerance, absorption, metabolism, distribution, excretion and, if possible, to gain an early understanding of its effectiveness.
- Phase II: The biological product is administered to a limited patient population to identify possible adverse effects and safety risks, to preliminarily evaluate the efficacy of the product for specific targeted diseases and to determine dosage tolerance and optimal dosage.
- Phase III The biological product is administered to an expanded patient population in adequate and well-controlled clinical trials to generate sufficient data to statistically confirm the potency and safety of the product for approval, to establish the overall risk-benefit profile of the product and to provide adequate information for the labelling of the product.

Progress reports detailing the results of the clinical trials must be submitted at least annually to FDA and more frequently if serious adverse events occur. The FDA or the sponsor may suspend or terminate a clinical trial at any time on various grounds, including a finding that the research subjects are being exposed to an unacceptable health risk. Similarly, an IRB can suspend or terminate approval of a clinical trial at its institution if the clinical trial is not being conducted in accordance with the IRB's requirements or if the biologic has been associated with unexpected serious harm to patients.

## ***FDA Guidance Governing Gene Therapy Products***

The FDA has issued various guidance documents regarding gene therapies, which outline additional factors that FDA will consider at each of the above stages of development and relate to, among other things, the proper preclinical assessment of gene therapies; the chemistry, manufacturing, and control information that should be included in an IND application; the proper design of tests to measure product potency in support of an IND or BLA application; and measures to observe delayed adverse effects in subjects who have been exposed to investigational gene therapies when the risk of such effects is high.

If a gene therapy trial is conducted at, or sponsored by, institutions receiving the NIH funding for recombinant DNA research, a protocol and related documentation must be submitted to, and the study registered with, the NIH Office of Biotechnology Activities, or OBA, pursuant to the NIH Guidelines for Research Involving Recombinant DNA Molecules prior to the submission of an IND to FDA. In addition, many companies and other institutions not otherwise subject to the NIH Guidelines voluntarily follow them. The NIH will convene the Recombinant DNA Advisory Committee (RAC), a federal advisory committee, to discuss protocols that raise novel or particularly important scientific, safety or ethical considerations at one of its quarterly public meetings. The OBA will notify FDA of the RAC's decision regarding the necessity for full public review of a gene therapy protocol. RAC proceedings and reports are posted to the OBA web site and may be accessed by the public.

## ***Compliance with cGMP Requirements***

Manufacturers of biologics must comply with applicable cGMP regulations, including quality control and quality assurance and maintenance of records and documentation. Manufacturers and others involved in the manufacture and distribution of such products must also register their establishments with FDA and certain state agencies. Both domestic and foreign manufacturing establishments must register and provide additional information to FDA upon their initial participation in the manufacturing process. Establishments may be subject to periodic unannounced inspections by government authorities to ensure compliance with cGMPs and other laws. Discovery of problems may result in a government entity placing restrictions on a product, manufacturer, or holder of an approved BLA, and may extend to requiring withdrawal of the product from the market. The FDA will not approve an application unless it determines that the manufacturing processes and facilities are in compliance with cGMP requirements and adequate to assure consistent production of the product within required specification.

## ***Submission of a BLA***

The results of the preclinical and clinical studies, together with detailed information relating to the product's chemistry, manufacture, controls and proposed labeling, among other things, are submitted to FDA as part of a BLA requesting a license to market the product for one or more indications. Under federal law, the submission of most BLAs is subject to an application user fee, currently exceeding \$2.1 million, and the sponsor of an approved BLA is also subject to annual product and establishment user fees, currently exceeding \$104,000 per product and \$554,600 per establishment. These fees are typically increased annually. The FDA has agreed to specified performance goals in the review of BLAs. Most such applications are meant to be reviewed within ten months from the date of filing, and most applications for "priority review" products are meant to be reviewed within six months of filing.

The FDA may also refer applications to an advisory committee for review and a vote on approval. Typically, an advisory committee includes clinicians and other experts who review, evaluate and vote on a recommendation as to whether the application should be approved. The FDA is not bound by the recommendations of an advisory committee, but it considers such recommendations carefully when making decisions.

### ***Expedited Review***

The FDA is authorized to expedite the review of BLAs in several ways. Under the fast track program, the sponsor of a biologic candidate may request FDA to designate the product for a specific indication as a fast track product concurrent with or after the filing of the IND for the product candidate. In addition to other benefits, such as the ability to use surrogate endpoints and have greater interactions with FDA, FDA may initiate review of sections of a fast track product's BLA before the application is complete. FDA may also take certain actions with respect to products designated as breakthrough therapies, including holding meetings with the sponsor and the review team throughout the development process; providing timely advice to and communication with the product sponsor regarding development and approval; involving more senior staff in the review process; assigning a cross-disciplinary project lead for the review team; and taking certain steps to design the clinical trials in an efficient manner.

### ***FDA's Decision on a BLA and Post-Approval Requirements***

On the basis of the BLA and accompanying information, including the results of the inspection of the manufacturing facilities, FDA may issue an approval letter or a complete response letter. An approval letter authorizes commercial marketing of the biological product with specific prescribing information for specific indications. A complete response letter generally outlines the deficiencies in the submission and may require substantial additional testing or information in order for FDA to reconsider the application. If and when those deficiencies have been addressed to FDA's satisfaction in a resubmission of the BLA, FDA will issue an approval letter.

If FDA approves a product, it may limit the approved indications for use of the product, require that contraindications, warnings or precautions be included in the product labeling, require that post-approval studies, including Phase IV clinical trials, be conducted to further assess a biologic's safety after approval, require testing and surveillance programs to monitor the product after commercialization, or impose other conditions, including distribution restrictions or other risk management mechanisms, including Risk Evaluation and Mitigation Strategies (REMS). The FDA may prevent or limit further marketing of a product based on the results of post-market studies or surveillance programs.

Following approval, some types of changes to the approved product, such as adding new indications, manufacturing changes and additional labeling claims, are subject to further testing requirements and FDA review and approval. The product may also be subject to official lot release, meaning that the manufacturer is required to perform certain tests on each lot of the product before it is released for distribution. Other post-approval requirements include reporting of cGMP deviations that could affect the identity, potency, purity and overall safety of a distributed product, reporting of adverse effects, reporting new information regarding safety and efficacy, maintaining adequate record-keeping, and complying with electronic record and signature requirements.

### ***Biosimilars and Exclusivity***

The 2010 Patient Protection and Affordable Care Act authorized FDA to approve biosimilars. Under the Act, a manufacturer may submit an application for licensure of a biologic product that is "biosimilar to" or "interchangeable with" a previously approved biological product or "reference product." In order for FDA to approve a biosimilar product, it must find that there are no clinically meaningful differences between the reference product and proposed biosimilar product. A finding of "interchangeability" requires that a product is determined to be biosimilar to the reference product, and that the product can be expected to produce the same clinical results as the reference product. An application for a biosimilar product may not be submitted to FDA until four years following approval of the reference product, and it may not be approved until 12 years thereafter. These exclusivity provisions only apply to biosimilar companies and not companies that rely on their own data and file a full BLA.



### **Orphan Drug Exclusivity**

Under the Orphan Drug Act, FDA may designate a biological product as an "orphan drug" if it is intended to treat a rare disease or condition (generally meaning that it affects fewer than 200,000 individuals in the United States, or more in cases in which there is no reasonable expectation that the cost of developing and making a biological product available in the United States for treatment of the disease or condition will be recovered from sales of the product). If a product with orphan status receives the first FDA approval, it will be granted 7 years of market exclusivity (meaning that FDA may not approve any other applications for the same product for the same indication for seven years, except in certain limited circumstances). Competitors may receive approval of different products for the indication for which the orphan product has exclusivity and may obtain approval for the same product but for a different indication. Orphan product designation does not convey any advantage in or shorten the duration of the regulatory review and approval process. In the European Union, we have been granted orphan drug exclusivity for Glybera for treatment of LPLD until October 2022, subject to the conditions applicable to orphan drug exclusivity. The FDA has also granted orphan drug designation to Glybera for treatment of LPLD, meaning that it will receive orphan drug exclusivity if it is the first product approved for that indication.

### **Pediatric Exclusivity**

Pediatric exclusivity is another type of regulatory exclusivity in the United States and, if granted, provides for the attachment of an additional six months of marketing protection to the term of any existing regulatory exclusivity, including orphan exclusivity and exclusivity against biosimilars. This six-month exclusivity may be granted if a BLA sponsor submits pediatric data that fairly respond to a written request from the FDA for such data. The data do not need to show the product to be effective in the pediatric population studied; rather, if the clinical trial is deemed to fairly respond to the FDA's request, the additional protection is granted. If reports of requested pediatric studies are submitted to and accepted by FDA within the statutory time limits, whatever statutory or regulatory periods of exclusivity cover the product are extended by six months.

### **FDA Regulation of Companion Diagnostics**

We may seek to develop *in vitro* and *in vivo* companion diagnostics for use in selecting the patients that we believe will respond to our gene therapies. FDA officials have issued draft guidance that, when finalized, would address issues critical to developing *in vitro* companion diagnostics, such as biomarker qualification, establishing clinical validity, the use of retrospective data, the appropriate patient population and when FDA will require that the device and the drug be approved simultaneously. The draft guidance issued in July 2011 states that if safe and effective use of a therapeutic product depends on an *in vitro* diagnostic, then FDA generally will require approval or clearance of the diagnostic at the same time that FDA approves the therapeutic product. The FDA has yet to issue further guidance, and it is unclear whether it will do so, or what the scope would be.

### **Anti-Kickback Provisions and Requirements**

The federal anti-kickback statute prohibits, among other things, knowingly and willfully offering, paying, soliciting or receiving remuneration to induce or in return for purchasing, leasing, ordering or arranging for the purchase, lease or order of any healthcare item or service reimbursable under Medicare, Medicaid or other federally financed healthcare programs. This statute has been interpreted to apply to arrangements between pharmaceutical manufacturers on the one hand and prescribers, purchasers and formulary managers on the other. Violations of the anti-kickback statute are punishable by imprisonment, criminal fines, civil monetary penalties and exclusion from participation in federal healthcare programs. Although there are a number of statutory exemptions and regulatory safe harbors protecting certain common activities from prosecution or other regulatory sanctions, the exemptions and safe harbors are drawn narrowly, and practices that involve remuneration intended to induce prescribing, purchases or recommendations may be subject to scrutiny if they do not qualify for an exemption or safe harbor.

Federal false claims laws prohibit any person from knowingly presenting, or causing to be presented, a false claim for payment to the federal government, or knowingly making, or causing to be made, a false statement to have a false claim paid. Pharmaceutical and other healthcare companies have been prosecuted under these laws for allegedly inflating drug prices they report to pricing services, which in turn were used by the government to set Medicare and Medicaid reimbursement rates, and for allegedly providing free product to customers with the expectation that the customers would bill federal programs for the product. In addition, certain marketing practices, including off-label promotion, have also been alleged by government agencies to violate false claims laws. The majority of states also have statutes or regulations similar to the federal anti-kickback law and false claims laws, which apply to items and services reimbursed under Medicaid and other state programs, or, in several states, apply regardless of the payor.

### ***Coverage, Pricing and Reimbursement***

The containment of healthcare costs has become a priority of federal, state and foreign governments, and the prices of drugs have been a focus in this effort. Third party payors are also increasingly challenging the prices charged for medical products and services and examining the medical necessity and cost-effectiveness of medical products and services, in addition to their safety and efficacy. If these third party payors do not consider a product to be cost-effective compared to other available therapies, they may not cover the product after approval as a benefit under their plans or, if they do, the level of payment may not be sufficient to allow a company to sell its products at a profit. The U.S. government, state legislatures and foreign governments have shown significant interest in implementing cost containment programs to limit the growth of government-paid health care costs, including price controls, restrictions on reimbursement and requirements for substitution of generic products for branded prescription drugs. Adoption of such controls and measures, and tightening of restrictive policies in jurisdictions with existing controls and measures, could limit payments for pharmaceuticals.

As a result, the marketability of any product which receives regulatory approval for commercial sale may suffer if the government and third party payors fail to provide adequate coverage and reimbursement. In addition, an increasing emphasis on managed care in the United States has increased and will continue to increase the pressure on drug pricing. Coverage policies, third party reimbursement rates and drug pricing regulation may change at any time. In particular, the Patient Protection and Affordable Care Act contains provisions that may reduce the profitability of drug products, including, for example, increased rebates for drugs sold to Medicaid programs, extension of Medicaid rebates to Medicaid managed care plans, mandatory discounts for certain Medicare Part D beneficiaries and annual fees based on pharmaceutical companies' share of sales to federal health care programs. Even if favorable coverage and reimbursement status is attained for one or more products that receive regulatory approval, less favorable coverage policies and reimbursement rates may be implemented in the future.

### ***Regulation in the European Union***

Product development, the regulatory approval process, and safety monitoring of medicinal products and their manufacturers in the European Union proceed in much the same manner as they do in the United States. Therefore, many of the issues discussed above apply similarly in the context of the European Union. In addition, drugs are subject to the extensive price and reimbursement regulations of the various European Union member states.

#### ***Clinical trials***

As is the case in the United States, the various phases of preclinical and clinical research in the European Union are subject to significant regulatory controls. The Clinical Trials Directive 2001/20/EC, as amended, provides a system for the approval of clinical trials in the European Union via implementation through national legislation of the member states. Under this system, approval must be obtained from the competent national authority of a European Union member state in which the clinical trial is to be

conducted. Furthermore, a clinical trial may only be started after a competent ethics committee has issued a favorable opinion on the clinical trial application, which must be supported by an investigational medicinal product dossier with supporting information prescribed by the Clinical Trials Directive and corresponding national laws of the member states and further detailed in applicable guidance documents. The sponsor of a clinical trial, or its legal representative, must be based in the European Economic Area. European regulators and ethics committees also require the submission of adverse event reports during a study and a copy of the final study report.

### *Marketing approval*

Marketing approvals under the European Union regulatory system may be obtained through a centralized or decentralized procedure. The centralized procedure results in the grant of a single marketing authorization that is valid for all—currently 28—European Union member states.

Pursuant to Regulation (EC) No 726/2004, as amended, the centralized procedure is mandatory for drugs developed by means of specified biotechnological processes, advanced therapy medicinal products as defined in Regulation (EC) No 1394/2007, drugs for human use containing a new active substance for which the therapeutic indication is the treatment of specified diseases, including but not limited to acquired immune deficiency syndrome, neurodegenerative disorders, auto-immune diseases and other immune dysfunctions, as well as drugs designated as orphan drugs pursuant to Regulation (EC) No 141/2000. The Committee for Medicinal Products for Human Use, or CHMP, established within the EMA also has the discretion to permit other products to use the centralized procedure if it considers them sufficiently innovative or they contain a new active substance. Given our focus on gene therapies, which fall within the category of advanced therapy medicinal products, or ATMPs, and orphan indications, our products and product candidates should typically qualify for the centralized procedure.

In the marketing authorization application, or MAA, the applicant has to properly and sufficiently demonstrate the quality, safety and efficacy of the drug. Under the centralized approval procedure, the CHMP, is responsible for drawing up the opinion of the EMA on any matter concerning the admissibility of the files submitted in accordance with the centralized procedure, such as an opinion on the granting, variation, suspension or revocation of a marketing authorization, and pharmacovigilance. For ATMPs, a special scientific committee within the EMA known as the Committee for Advanced Therapies, or CAT, is responsible in conjunction with the CHMP for the evaluation of ATMPs. The CAT is primarily responsible for the scientific evaluation of ATMPs and prepares a draft opinion on the quality safety and efficacy of each ATMP for which a MAA is submitted. The CAT's opinion is then taken into account by the CHMP when giving its final recommendation regarding the authorization of a product in view of the balance of benefits and risks identified. Although the CAT's draft opinion is submitted to the CHMP for final approval, the CHMP may depart from the draft opinion, if it provides detailed scientific justification.

The CHMP and CAT are also responsible for providing guidelines on ATMPs and have published numerous guidelines, including specific guidelines on gene therapies. These guidelines provide additional guidance on the factors that the EMA will consider in relation to the development and evaluation of ATMPs and include, among other things, the preclinical studies required to characterize ATMPs; the manufacturing and control information that should be submitted in a MAA; and post-approval measures required to monitor patients and evaluate the long term efficacy and potential adverse reactions of ATMPs. Although these guidelines are not legally binding, we believe that our compliance with them is likely necessary to gain approval for any of our product candidates.

The maximum timeframe for the evaluation of an MAA by the CHMP under the centralized procedure is 210 days after receipt of a valid application. This period will be suspended until such time as the supplementary information requested by the CHMP, or in the case of ATMPs information also requested by the CAT, has been provided by the applicant. Likewise, this time-limit will be suspended for the time allowed for the applicant to prepare oral or written explanations. When an application is submitted for a

marketing authorization in respect of a drug which is of major interest from the point of view of public health and in particular from the viewpoint of therapeutic innovation, the applicant may request an accelerated assessment procedure. If the CHMP accepts such request, the time-limit of 210 days will be reduced to 150 days but it is possible that the CHMP can revert to the standard time-limit for the centralized procedure if it considers that it is no longer appropriate to conduct an accelerated assessment.

If the CHMP concludes that the quality, safety and efficacy of the product is sufficiently proven, it adopts a positive opinion. This is sent to the European Commission which drafts a decision. After consulting with the member states, the European Commission adopts a decision and grants a marketing authorization, which is valid for the whole of the European Union.

The European Commission may grant a so-called "marketing authorization under exceptional circumstances". Such authorization is intended for products for which the applicant can demonstrate that it is unable to provide comprehensive data on the efficacy and safety under normal conditions of use, because the indications for which the product in question is intended are encountered so rarely that the applicant cannot reasonably be expected to provide comprehensive evidence, or in the present state of scientific knowledge, comprehensive information cannot be provided, or it would be contrary to generally accepted principles of medical ethics to collect such information. Consequently, marketing authorization under exceptional circumstances may be granted subject to certain specific obligations, which may include the following:

- the applicant must complete an identified programme of studies within a time period specified by the competent authority, the results of which form the basis of a reassessment of the benefit/risk profile;
- the medicinal product in question may be supplied on medical prescription only and may in certain cases be administered only under strict medical supervision, possibly in a hospital and in the case of a radio-pharmaceutical, by an authorised person; and
- the package leaflet and any medical information must draw the attention of the medical practitioner to the fact that the particulars available concerning the medicinal product in question are as yet inadequate in certain specified respects.

A marketing authorization under exceptional circumstances is subject to annual review to reassess the risk-benefit balance in an annual re-assessment procedure. Continuation of the authorization is linked to the annual reassessment and a negative assessment could potentially result in the marketing authorization being suspended or revoked. The renewal of a marketing authorization of a medicinal product under exceptional circumstances, however, follows the same rules as a "normal" marketing authorization. Thus, a marketing authorization under exceptional circumstances is granted for an initial 5 years, after which the authorization will become valid indefinitely, unless the EMA decides that safety grounds merit one additional five-year renewal.

The European Union also provides for a system of regulatory data and market exclusivity. According to Article 14(11) of Regulation (EC) No 726/2004, as amended, and Article 10(1) of Directive 2001/83/EC, as amended, upon receiving marketing authorization, new chemical entities approved on the basis of complete independent data package benefit from eight years of data exclusivity and an additional two years of market exclusivity. Data exclusivity prevents regulatory authorities in the European Union from referencing the innovator's data to assess a generic (abbreviated) application. During the additional two-year period of market exclusivity, a generic marketing authorization can be submitted, and the innovator's data may be referenced, but no generic medicinal product can be marketed until the expiration of the market exclusivity. The overall ten-year period will be extended to a maximum of eleven years if, during the first eight years of those ten years, the marketing authorization holder obtains an authorization for one or more new therapeutic indications which, during the scientific evaluation prior to their authorization, are held to bring a significant clinical benefit in comparison with existing therapies. Even if a compound is considered to be a new chemical entity and the innovator is able to gain the period of data exclusivity, another

company nevertheless could also market another version of the drug if such company obtained marketing authorization based on an MAA with a complete independent data package of pharmaceutical test, pre-clinical tests and clinical trials.

Additional rules apply to medicinal products for pediatric use under Regulation (EC) No 1901/2006. Potential incentives include a six-month extension of any supplementary protection certificate granted pursuant to Regulation (EC) No 469/2009, however not in cases in which the relevant product is designated as orphan medicinal products pursuant to Regulation (EC) No 141/2000, as amended. Instead, medicinal products designated as orphan medicinal product may enjoy an extension of the ten-year market exclusivity period granted under Regulation (EC) No 141/2000 to twelve years subject to the conditions applicable to orphan drugs.

#### *Manufacturing and manufacturers' license*

Pursuant to Directive 2003/94/EC as transposed into the national laws of the member states, the manufacturing of investigational medicinal products and approved drugs is subject to a separate manufacturer's license and must be conducted in strict compliance with cGMP requirements, which mandate the methods, facilities, and controls used in manufacturing, processing, and packing of drugs to assure their safety and identity. Manufacturers must have at least one qualified person permanently and continuously at their disposal. The qualified person is ultimately responsible for certifying that each batch of finished product released onto the market has been manufactured in accordance with cGMP and the specifications set out in the marketing authorization or investigational medicinal product dossier. cGMP requirements are enforced through mandatory registration of facilities and inspections of those facilities. Failure to comply with these requirements could interrupt supply and result in delays, unanticipated costs and lost revenues, and subject the applicant to potential legal or regulatory action, including but not limited to warning letters, suspension of manufacturing, seizure of product, injunctive action or possible civil and criminal penalties.

#### *Advertising*

In the European Union, the promotion of prescription medicines is subject to intense regulation and control, including a prohibition on direct-to-consumer advertising. All medicines advertising must be consistent with the product's approved summary of products characteristics, factual, accurate, balanced and not misleading. Advertising of medicines pre-approval or off-label. Some jurisdictions require that all promotional materials for prescription medicines be subjected to either prior internal or regulatory review and approval.

#### *Other Regulatory Requirements*

A holder of a marketing authorization for a medicinal product is legally obliged to fulfill a number of obligations by virtue of its status as a marketing authorization holder, or MAH. The MAH can delegate the performance of related tasks to third parties, such as distributors or marketing partners, provided that this delegation is appropriately documented and the MAH maintains legal responsibility and liability.

The obligations of an MAH include:

- **Manufacturing and Batch Release.** MAHs should guarantee that all manufacturing operations comply with relevant laws and regulations, applicable good manufacturing practices, with the product specifications and manufacturing conditions set out in the marketing authorization and that each batch of product is subject to appropriate release formalities.
- **Pharmacovigilance.** MAHs are obliged to establish and maintain a pharmacovigilance system, including a qualified person responsible for oversight. submit safety reports to the regulators and comply with the good pharmacovigilance practice guidelines adopted by the EMA.

- *Advertising and Promotion.* MAH holders remain responsible for all advertising and promotion of its products, including promotional activities by other companies or individuals on their behalf and in some cases must conduct internal or regulatory pre-approval of promotional materials.
- *Medical Affairs/Scientific Service.* MAHs are required to disseminate scientific and medical information on its medicinal products to healthcare professionals, regulators and patients.
- *Legal Representation and Distributor Issues.* MAHs are responsible for regulatory actions or inactions of their distributors and agents.
- *Preparation, Filing and Maintenance of the Application and Subsequent Marketing Authorization.* MAHs must maintain appropriate records, comply with the marketing authorization's terms and conditions, fulfill reporting obligations to regulators, submit renewal applications and pay all appropriate fees to the authorities.

We hold the marketing authorization under exceptional circumstances granted for Glybera and we may hold any future marketing authorizations granted for our product candidates in our own name, or appoint an affiliate or a collaboration partner to hold marketing authorizations on our behalf. Any failure by an MAH to comply with these obligations may result in regulatory action against an MAH and ultimately threaten our ability to commercialize our products.

### *Reimbursement*

In the European Union, the pricing and reimbursement mechanisms by private and public health insurers vary largely by country and even within countries. In respect of the public systems reimbursement for standard drugs is determined by guidelines established by the legislator or responsible national authority. The approach taken varies from member state to member state. Some jurisdictions operate positive and negative list systems under which products may only be marketed once a reimbursement price has been agreed. Other member states allow companies to fix their own prices for medicines, but monitor and control company profits and may limit or restrict reimbursement. The downward pressure on healthcare costs in general, particularly prescription drugs, has become very intense. As a result, increasingly high barriers are being erected to the entry of new products and some of EU countries require the completion of studies that compare the cost-effectiveness of a particular product candidate to currently available therapies in order to obtain reimbursement or pricing approval. Special pricing and reimbursement rules may apply to orphan drugs. Inclusion of orphan drugs in reimbursement systems tend to focus on the medical usefulness, need, quality and economic benefits to patients and the healthcare system as for any drug. Acceptance of any medicinal product for reimbursement may come with cost, use and often volume restrictions, which again can vary by country. In addition, results based rules of reimbursement may apply.

### *Orphan Drug Regulation*

In the European Union, Regulation (EC) No 141/2000, as amended, states that a drug will be designated as an orphan drug if its sponsor can establish:

- that it is intended for the diagnosis, prevention or treatment of a life-threatening or chronically debilitating condition affecting not more than five in ten thousand persons in the Community when the application is made, or that it is intended for the diagnosis, prevention or treatment of a life-threatening, seriously debilitating or serious and chronic condition in the European Union and that without incentives it is unlikely that the marketing of the drug in the European Union would generate sufficient return to justify the necessary investment; and
- that there exists no satisfactory method of diagnosis, prevention or treatment of the condition in question that has been authorized in the European Union or, if such method exists, that the drug will be of significant benefit to those affected by that condition.

Regulation (EC) No 847/2000 sets out further provisions for implementation of the criteria for designation of a drug as an orphan drug. An application for the designation of a drug as an orphan drug must be submitted at any stage of development of the drug before filing of a marketing authorization application.

If a European Union-wide community marketing authorization in respect of an orphan drug is granted pursuant to Regulation (EC) No 726/2004 or if all the European Union member states have granted marketing authorizations in accordance with the procedures for mutual recognition, the European Union and the member states will not, for a period of 10 years, accept another application for a marketing authorization, or grant a marketing authorization or accept an application to extend an existing marketing authorization, for the same therapeutic indication, in respect of a similar drug. This period may however be reduced to six years if, at the end of the fifth year, it is established, in respect of the drug concerned, that the criteria for orphan drug designation are no longer met, in other words, when it is shown on the basis of available evidence that the product is sufficiently profitable not to justify maintenance of market exclusivity. Notwithstanding the foregoing, a marketing authorization may be granted, for the same therapeutic indication, to a similar drug if:

- the holder of the marketing authorization for the original orphan drug has given its consent to the second applicant;
- the holder of the marketing authorization for the original orphan drug is unable to supply sufficient quantities of the drug; or
- the second applicant can establish in the application that the second drug, although similar to the orphan drug already authorized, is safer, more effective or otherwise clinically superior.

Regulation (EC) No 847/2000 lays down definitions of the concepts 'similar drug' and 'clinical superiority'. Other incentives available to orphan drugs in the European Union include financial incentives such as a reduction of fees or fee waivers and protocol assistance. Orphan drug designation does not shorten the duration of the regulatory review and approval process.

### ***Regulation in Other Countries***

For other countries outside of the United States and the European Union the requirements governing the development and approval process as well as post-approval and pricing and reimbursement requirements vary from country to country. In general, clinical studies are to be conducted in accordance with GCP and the applicable regulatory requirements and the ethical principles originating from the Declaration of Helsinki.

If we fail to comply with applicable foreign regulatory requirements, we may be subject to, among other things, fines, suspension or withdrawal of regulatory approvals, product recalls, seizure of products, operating restrictions and criminal prosecution.

### **History of uniQure**

Our business was founded in 1998 by scientists who were investigating LPLD at the Academic Medical Center of the University of Amsterdam, or AMC. In our early years we received funding and subsidized rent from the AMC, government grants, income for cGMP contract manufacturing of biologics for third parties, and small amounts of equity financing. Since our first institutional venture capital financing in 2006, we have funded our operations primarily through private and public placements of equity securities and convertible debt securities. Our predecessor entity, Amsterdam Molecular Therapeutics (AMT) N.V., or AMT, completed an initial public offering of its ordinary shares on Euronext Amsterdam in 2007 and subsequently delisted from that exchange in 2012. uniQure acquired the business of AMT in the first half of 2012.

## **Facilities**

Our headquarters and principal laboratories are located at Meibergdreef in Amsterdam, the Netherlands, which we lease from the AMC. This 25,932 square-foot location also houses our manufacturing facility, which the EMA has approved for clinical and commercial grade production. The lease for this facility terminates in 2016. We also have a leased facility in Lexington, Massachusetts, where we have begun the build-out of a 53,000 square foot manufacturing facility. The lease for this facility terminates in 2024, and subject to the provisions of the lease, may be renewed for two subsequent five year terms.

## **Legal Proceedings**

We are not involved in any material legal proceedings.

On December 11, 2013, we received a formal request for arbitration from Extera Partners, a consulting firm based in Cambridge, Massachusetts, alleging a fee to be due in respect of consulting services provided to us in connection with a partnering transaction. The amount claimed is \$100,000 plus 2.5% of all proceeds, including equity investments, we receive from Chiesi pursuant to our collaboration agreements entered into in the second quarter of 2013. Our engagement letter with Extera Partners contains a cap limiting the maximum payment to €5.0 million. We have reviewed this claim with counsel and believe that the claim is without merit. We intend to vigorously defend against it.

## **Employees**

As of September 30, 2013, we had a total of 79 employees, of whom 27 had an M.D. or Ph.D. degree, or the foreign equivalent. Of these employees, 19 were engaged in research and development, six in clinical development, and two in business development functions. We also engaged 33 consultants and contract workers. We do not currently have in place a works council. We believe that our relations with our employees are good.



## MANAGEMENT

### General

We have a two-tier board structure consisting of our management board (*raad van bestuur*) and a separate supervisory board (*raad van commissarissen*). Below is a summary of relevant information concerning our supervisory board, management board and senior management, as well as a brief summary of certain significant provisions of Dutch corporate law, the articles of association that will be in effect upon the closing of this offering and the Dutch Corporate Governance Code, or DCGC, in respect of our management board and supervisory board.

### Members of Our Supervisory Board, Management Board and Senior Management

#### Supervisory board

The following table sets forth information with respect to each of our supervisory board members and their respective ages as of the date of this prospectus. The terms of office of all our supervisory board members expire according to a rotation plan drawn up by our supervisory board. The business address of our supervisory board members is our registered office address at Meibergdreef 61, Amsterdam 1105 BA, the Netherlands.

Our supervisory board is currently composed of the following members, all of whom will be independent under applicable NASDAQ standards immediately following the closing of this offering:

NAME	AGE	POSITION	MEMBER SINCE <sup>(1)</sup>
Ferdinand Verdonck	71	Member of the Supervisory Board (Chairman)	July 2012
Sander Slootweg	45	Member of the Supervisory Board	April 2012
Sander van Deventer	59	Member of the Supervisory Board	April 2012
Joseph M. Feczko	64	Member of the Supervisory Board	April 2012
François Meyer	65	Member of the Supervisory Board	April 2012
Paula Soteropoulos	46	Member of the Supervisory Board	July 2013

<sup>(1)</sup> For periods prior to 2012, certain of our directors served as directors of AMT, our predecessor entity.

**Ferdinand Verdonck** has served as our chairman since July 2012 and served as chairman of the AMT supervisory board from April 2007 until July 2012. He is a director on the boards of J.P. Morgan European Investment Trust, Groupe SNEF, Laco Information Services and Virtus Funds. From 1992 to 2003, he was the managing director of Almanij NV, a financial services company which has since merged with KBC and his responsibilities included company strategy, financial control, supervision of executive management and corporate governance, including board participation in publicly-traded and privately-held companies in many countries. He served as a member of the board of directors and chairman of the audit committee of two biotechnology companies in Belgium, Movetis and Galapagos. He has previously served as chairman of Banco Urquijo, a director of Dictaphone Corporation and a director of the Dutch Chamber of Commerce for Belgium and Luxembourg, member of the General Council and chairman of the audit committee of the Vlerick Leuven Ghent Management School. Mr. Verdonck holds a law degree from KU Leuven and degrees in economics from KU Leuven and the University of Chicago. We believe that Mr. Verdonck is qualified to serve on our supervisory board due to his expertise in the financial services and manufacturing industries and his service on the boards of directors of other companies.

**Sander Slootweg** has served as a member of our supervisory board since April 2012 and served as member of the AMT supervisory board from September 2006 to April 2008, including as Chairman from 2006 to 2007. Mr. Slootweg is a managing partner at Forbion Capital Partners, the Netherlands, a venture capital firm he co-founded in 2006. He currently serves on the boards of Forbion's portfolio companies

Xention, Ltd, Pulmagen Therapeutics, Ltd, Dezima Pharma, B.V. (Chairman), Ario Pharma Ltd. and Oxyrane, Ltd. In addition, in recent years Mr. Slootweg has served on the boards of Argenta Discovery Ltd (sold to Galapagos in 2010), Alantos Pharmaceuticals, Inc. (sold to Amgen in 2007), BioVex Group, Inc. (sold to Amgen in 2011), Impella Cardiosystems AG (sold to Abiomed, Inc. in 2005), Glycart AG (sold to Roche in 2005), Cambridge Drug Discovery Ltd (sold to Biofocus Plc in 2001), Fovea Pharmaceuticals S.A. (sold to Sanofi-Aventis in 2009) and Pieris AG. Mr. Slootweg holds degrees in Business and Financial Economics from the Free University of Amsterdam and in Business Administration from Nijenrode University, The Netherlands. We believe that Mr. Slootweg is qualified to serve on our supervisory board due to his expertise in the healthcare technology industry and his service on the boards of directors of other companies.

**Sander van Deventer** has served as a member of our supervisory board since April 2012 and served as member of the AMT supervisory board from April 2010 to April 2012. Dr. van Deventer was one of our co-founders and currently chairs uniQure's Scientific Advisory Board. He served as our interim Chief Executive Officer from January to October 2009. He is Professor of Translational Gastroenterology at the Leiden University Medical Center since 2008 and a partner of Forbion Capital Partners, which he joined in 2006. He serves on the boards of Cardoz AS, Argos Biotherapeutics, glCare Pharma Inc and Hookipa Biotech. He was previously a professor, head of the department of experimental medicine and chairman of the department of gastroenterology of the Academic Medical Center at the University of Amsterdam from 2002 to 2004, and subsequently professor of experimental medicine at the University of Amsterdam Medical School until 2008. He has more than 15 years of experience in biotechnology product development. He is the author of more than 350 scientific articles in peer-reviewed journals, and he serves as an advisor to regulatory authorities including the EMA and FDA. Dr. van Deventer holds a degree in medicine as well as a Ph.D. from the University of Amsterdam. We believe that Dr. van Deventer is qualified to serve on our supervisory board due to his expertise in the biotechnology industry and his service on the boards of directors of other biotechnology companies.

**Joseph M. Feczko** has served as a member of our supervisory board since April 2012 and served as a member of the AMT supervisory board from August 2010 to April 2012. Dr. Feczko worked for Pfizer Inc. from 1982 to 1992 and from 1996 to 2009, where he held positions of increasing responsibility in clinical research, regulatory affairs and safety culminating in the role of Senior Vice President and Chief Medical Officer. From 1992 to 1996, Dr. Feczko was Medical Director for GlaxoSmithKline R&D in the United Kingdom. Dr. Feczko is chairman of the board of directors at Cardoz Pharmaceuticals AB, and a director of Keryx Biopharmaceuticals, Inc. and ChemoCentryx Inc., as well as a member of the supervisory board of Cytheris. He is also a member of the board of directors of Accordia Global Health Foundation Research!America, and the Foundation of National Institute of Health, and a trustee of the New York Academy of Medicine. Dr. Feczko is a member of the Technical Expert Committee for the International Trachoma Initiative of the Task Force for Global Health. Between 2006-2011 he was a member of the Governing Board of the Technology Strategy Board of the United Kingdom. Dr. Feczko is Board Certified in Internal Medicine and Infectious Diseases. Dr. Feczko holds a bachelor of science degree from Loyola University and an M.D. from the University of Illinois College of Medicine. We believe that Dr. Feczko is qualified to serve on our supervisory board due to his expertise in the pharmaceutical and biotechnology industries.

**François Meyer** has served as a member of our supervisory board since April 2012 and served as a member of the AMT supervisory board from July 2010 to April 2012. Dr. Meyer was until recently CEO and Chairman of the board of TxCell SA, a cell therapy company located in France, and of which he is currently Executive Chairman. Prior to this, he was CEO of Gencell, a fully owned gene therapy subsidiary of Aventis until 2006. He was senior vice president R&D at Aventis Pharma until 2002 and prior to that he led global research at Rhone Poulenc Rorer. In the earlier part of his career he held senior management positions at Sandoz and led the gene and cell therapy business. He was a member of the board of directors or the scientific advisory board of a number of biotech companies in the gene and cell therapy area including

Introgen Therapeutics, Inc., Gene Therapy Inc., Systemix, Inc. and Biotransplant, Inc. We believe that Dr. Meyer is qualified to serve on our supervisory board due to his expertise and insight in the biotechnology industry.

**Paula Soteropoulos** has served as a member of our supervisory board since July 2013. Ms. Soteropoulos is Senior Vice President and General Manager, Cardiometabolic Business and Strategic Alliances at Moderna Therapeutics, Inc. a position she had held since July 2013. Previously, Ms. Soteropoulos has worked at Genzyme Corporation, a biotechnology company, from 1992 to 2013, most recently as Vice President and General Manager, Cardiovascular, Rare Diseases. Ms. Soteropoulos holds a bachelor of science degree in chemical engineering and a master of science degree in chemical and biochemical engineering, both from Tufts University, and holds an executive management certificate from the University of Virginia, Darden Graduate School of Business Administration. We believe Ms. Soteropoulos is qualified to serve on our supervisory board due to her extensive experience in the biotechnology industry.

### **Management board**

The following table sets out information with respect to each of our management board members, their respective ages and their positions at uniQure as of the date of this prospectus. The business address of our management board members is our registered office address at Meibergdreef 61, Amsterdam 1105 BA, the Netherlands.

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NAME	AGE	POSITION	DATE OF APPOINTMENT
Jörn Aldag	54	Chief Executive Officer	October 4, 2009
Piers Morgan	47	Chief Financial Officer	December 1, 2009

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**Jörn Aldag** has served as our chief executive officer since he joined AMT, now uniQure, in October 2009. He has led our corporate development including the expansion of our gene therapy pipeline, the marketing authorization process with the EMA for Glybera and the recapitalization of AMT to form uniQure. Before joining our company he was instrumental in building Evotec AG, a drug discovery company listed on the Frankfurt Stock Exchange, serving as chief financial officer from 1997 to 2000 and as president and chief executive officer from 2001 to 2009. Prior to Evotec, Mr. Aldag served in various financial management positions at MAN AG, and as Business Director at Treuhandanstalt, the agency responsible for privatizing the East German economy after the German reunification. Mr. Aldag is Chairman of Molecular Partners AG, Zurich, Switzerland, and holds business degrees from the Harvard Business School (Advanced Management Program) and the European Business School. We believe that Mr. Aldag is qualified to serve on our management board due to his broad expertise in the biotechnology industry and his deep general management experience.

**Piers Morgan** has served as our chief financial officer since he joined AMT in December 2009. Mr. Morgan is currently chairman of the board and a member of the audit committee of Trino Therapeutics, a biotechnology company. He has more than 13 years of experience as chief financial officer of several biotechnology companies, including Phytopharm plc, BioAlliance Pharma SA, and Arrow Therapeutics Ltd. Prior to this period, he spent ten years in investment banking, working in mergers & acquisitions and equity capital markets with Close Brothers and Ernst & Young corporate finance. He qualified as a chartered accountant in London with PricewaterhouseCoopers. Mr. Morgan is Chairman of Trino Therapeutics Ltd and holds a degree in law and management studies from Cambridge University. We believe that Mr. Morgan is qualified to serve on our management board due to his expertise in the biotechnology industry and his accounting background.

## Senior management

Our management board is supported by our senior management team. The following table sets forth information with respect to each of the members of our senior management team, their respective ages and their positions as of the date of this prospectus. The business address of the members of our senior management is our registered office address at Meibergdreef 61, Amsterdam 1105 BA, the Netherlands.

NAME	AGE	POSITION
Philip Astley-Sparke	42	President, US Operations
Christian Meyer, M.D.	46	Chief Medical Officer
Harald Petry	54	Chief Science Officer
Hans Preusting	51	Chief Business Officer
Hans Christian Rohde	56	Chief Commercial Officer

**Philip Astley-Sparke** has served as the president of our United States operations since January 2013. Mr. Astley-Sparke has been a venture partner at Forbion Capital Partners, a venture capital fund, since May 2012. He served as vice president and general manager at Amgen, Inc., a biopharmaceutical company, until December 2011, following Amgen's acquisition of BioVex Group, Inc., a biotechnology company, in March 2011. Mr. Astley-Sparke had been president and chief executive officer of BioVex Group since 2007, which he joined in 2000, and previously served in the roles of President & COO and CFO. He oversaw the company's relocation to the U.S. where he grew operations from scratch, including overseeing the construction of a commercial-grade manufacturing facility. Prior to BioVex, Mr. Astley-Sparke was a healthcare investment banker with Chase H&Q/Robert Fleming. He qualified as a chartered accountant with Arthur Andersen in London and holds a bachelor's degree in cellular pathology and molecular pathology from Bristol University in the United Kingdom. He also serves as chairman of the board of Oxyrane, a biotechnology company.

**Christian Meyer, M.D.** has served as our chief medical officer since October 2013. Dr. Meyer has more than 13 years of clinical research experience with both biotechnology companies and large pharma, with particular expertise in the development of treatments for rare diseases, including acute intermittent porphyria and lysosomal storage disorders. From 2010-2013 he was the chief medical officer at Cardoz AB, a pharmaceutical company. Prior to that, from 2006 to 2010, Dr. Meyer held leadership positions in clinical development at Symphogen A/S, a biopharmaceutical company, where he was senior vice president for medical affairs and vice president of clinical development. Prior to Symphogen A/S, he played an important role in clinical development at Zymenex A/S and spent five years in clinical development at Novo Nordisk A/S, both biopharmaceutical companies. Dr. Meyer received both his M.D. and Ph.D. degrees from the University of Copenhagen, Denmark.

**Harald Petry** has served as our chief science officer since January 2012. Dr. Petry joined AMT in May 2007 as director of research and development. He has worked in the area of gene therapy for more than 15 years and has extensive experience in pharmaceutical research. Prior to joining us, he worked at Jenapharm GmbH (Germany), a pharmaceutical company, from 2001 to 2002 and Berlex Biosciences (US), a biotechnology company, from 2002 to 2007 in different functions with increasing managerial and leadership responsibility. Dr. Petry holds his doctoral degree in biology from Justus-Liebig-Universität Giessen.

**Hans Preusting** has served as our chief business officer since July 2011, still at AMT, where he first joined us as a Director of Process Development and Manufacturing, in August 2006. He holds a PhD in biochemistry and an MBA from Rotterdam School of Management. He has more than 20 years of experience in product development and manufacturing using fermentation and cell culture techniques. Prior to joining

us, he was at Solvay Pharmaceuticals, DSM and Gist-brocades. Dr. Preusting holds two patents and has published more than 20 scientific articles.

**Hans Christian Rohde** has served as our chief commercial officer since December 2012. Mr. Rohde has almost 25 years of experience in commercial roles at leading biotechnology and pharmaceutical companies. From 2007 until 2012 he was chief commercial officer at Basilea Pharmaceutica, a pharmaceutical company, and member of its executive management committee with responsibility for global commercial operations, marketing, supply chain, medical affairs, pricing and market access. Prior to Basilea Pharmaceutica, Mr. Rohde was corporate vice president, head of global therapeutic areas reproductive health and endocrinology at Merck-Serono, a pharmaceutical company, from 2003 until 2007. Before this he was responsible for international marketing and global market development at Biogen Idec, a biotechnology company. Mr Rohde holds a masters of science from the University of Copenhagen and a masters of business administration from the Birmingham Business School, the University of Birmingham in the UK.

## **Corporate Governance**

### ***Supervisory Board***

Our supervisory board is responsible for the supervision of the activities of our management board and our company's general affairs and business. Our supervisory board may also, on its own initiative, provide the management board with advice and may request any information from the management board that it deems appropriate. In performing its duties, the supervisory board is required to act in the interests of our company (including its stakeholders) and its associated business as a whole. The members of the supervisory board are not authorized to represent us in dealings with third parties.

The articles of association of our company provide that members of the supervisory board are appointed at the general meeting of shareholders following a non-binding proposal of the supervisory board. The number of supervisory board members is determined by the supervisory board itself.

Our articles of association provide that members of our supervisory board will serve for a maximum term of four years, unless the resolution appointing a supervisory board member provides otherwise, and may only be reappointed twice. The articles of association provide that the supervisory board members must retire periodically in accordance with a rotation plan to be adopted by the supervisory board. The supervisory board appoints a chairman from among its members if there is more than one supervisory director.

Under our articles of association, the general meeting of shareholders may suspend or dismiss supervisory board members at any time. A resolution by the general meeting of shareholders to suspend or dismiss a supervisory director requires at least a two thirds majority of the votes cast, provided such majority represents more than half the issued share capital, unless the proposal was made by the supervisory board, in which case a simple majority of the votes cast is sufficient.

Our supervisory board can only adopt resolutions by an absolute majority of the total number of votes to be cast if the majority of the supervisory board members then in office are present or represented. The supervisory board may also adopt resolutions outside a meeting, provided that such resolutions are adopted in writing and submitted to all members of the supervisory board and provided that no such supervisory board member objects to adopting resolutions without conducting a meeting. Each supervisory board member is entitled to cast one vote.

### ***Management Board***

Our management board is responsible for the day-to-day management of our operations under the supervision of the supervisory board. The management board is required to

- keep the supervisory board informed in a timely manner in order to allow the supervisory board to carry out its responsibilities;

- consult with the supervisory board on important matters; and
- submit certain important decisions to the supervisory board for its approval, as more fully described below.

Our management board may perform all acts necessary or useful for achieving our corporate purposes, other than those acts that are prohibited by law or by our articles of association, as more fully discussed below. The management board as a whole, or any two members acting jointly, is authorized to represent us in dealings with third parties.

Under our articles of association, the number of members of the management board is determined by the supervisory board and the management board must consist of at least one member.

Members of the management board are appointed by the general meeting of shareholders following a non-binding proposal of the supervisory board. Our articles of association provide that, unless the resolution appointing a management board member provides otherwise, members of our management board will serve for a maximum term of four years and may only be reappointed immediately following one term for a term of not more than four years at a time.

The general meeting of shareholders may suspend or dismiss members of the management board at any time. The supervisory board may also suspend members of the management board at any time. A suspension of a management board member by the supervisory board may be discontinued at any time by action by the general meeting of shareholders.

Under the Dutch Civil Code, decisions of our management board require approval by our general meeting of shareholders if and when these relate to an important change in the identity or character of the company or of our business. Such decisions include:

- a transfer of all or substantially all of our business to a third party;
- the entry into or termination of, by ourselves or one of our subsidiaries, a material long-term cooperation with another person or partnership or a general or limited partnership in which we serve as a general partner;
- the acquisition or divestment of an interest in the capital of another legal person or partnership as a participating holding (*deelneming*), within the meaning of the Dutch Civil Code, having a value of at least one-third of the aggregate amount of our assets according to our most recent consolidated annual balance sheet.

Under our articles of association, the following decisions of the management board must be approved by the supervisory board:

- the sale or disposition of all, or an essential part of, our assets;
- the issuance and acquisition of shares and of debentures chargeable against us or chargeable against a limited partnership (*commanditaire vennootschap*), or a general partnership (*vennootschap onder firma*) of which we are the fully liable partner;
- the application for quotation, or withdrawal of quotation, of our shares or debt on any stock exchange;
- our entry into or termination of any long-term, material cooperation by us or our subsidiary with another legal entity or partnership;
- our investment in the capital of another company in an amount equal to at least one-fourth of our issued capital plus our reserves, as reflected on our most recent balance sheet, as well as a material change to such investment;
- filing a petition for bankruptcy (*faillissement*) or for suspension of payments (*surseance van betaling*);
- the termination of a significant number of our employees simultaneously or within a short period of time;

- a significant change in the employment conditions of our employees; and
- a decrease in our issued capital.

Our supervisory board may determine that a resolution that would otherwise be subjected to its approval will not require such approval if the amount involved does not exceed a value fixed by the supervisory board and notice is given to the management board in writing. Our supervisory board may also require that additional actions, beyond those listed above, by the management board be conditioned upon the supervisory board's approval. Such actions must be clearly specified to the management board in writing. The absence of approval of the supervisory board does not affect the authority of the management board or its members to represent us in dealings with third parties.

The management board must inform the supervisory board in writing of the key elements of our strategic policy, our general and financial risks and our management and control system at least once a year.

### **Committees of the Supervisory Board**

Upon the completion of this offering, we will have an audit committee, a remuneration committee and a nominating and corporate governance committee. We have adopted a charter for each of these committees.

#### ***Audit Committee***

Upon the completion of this offering, our audit committee will consist of Messrs. Ferdinand Verdonck (Chairman), \_\_\_\_\_, and \_\_\_\_\_. Each member satisfies the independence requirements of the NASDAQ listing standards, and Mr. \_\_\_\_\_ qualifies as an "audit committee financial expert," as defined in Item 16A of Form 20-F and as determined by our supervisory board. The audit committee will oversee our accounting and financial reporting processes and the audits of our consolidated financial statements. The audit committee will be responsible for, among other things:

- making recommendations to our supervisory board regarding the appointment by the general meeting of shareholders of our independent auditors;
- overseeing the work of the independent auditors, including resolving disagreements between management and the independent auditors relating to financial reporting;
- pre-approving all audit and non-audit services permitted to be performed by the independent auditors;
- reviewing the independence and quality control procedures of the independent auditors;
- discussing material off-balance sheet transactions, arrangements and obligations with management and the independent auditors;
- reviewing and approving all proposed related-party transactions;
- discussing the annual audited consolidated and statutory financial statements with management;
- annually reviewing and reassessing the adequacy of our audit committee charter;
- meeting separately with the independent auditors to discuss critical accounting policies, recommendations on internal controls, the auditor's engagement letter and independence letter and other material written communications between the independent auditors and the management; and
- attending to such other matters as are specifically delegated to our audit committee by our supervisory board from time to time.

#### ***Remuneration Committee***

Upon the completion of this offering, our remuneration committee will consist of Messrs. Sander van Deventer, Joseph Feczko, and Francois Meyer. Each member satisfies the independence requirements of the NASDAQ listing standards. The remuneration committee will assist the supervisory board in reviewing and approving or recommending our compensation structure, including all forms of compensation relating to our supervisory directors and management. Members of our management may not be present at any committee

meeting while the compensation of our chief executive officer is deliberated. Subject to the terms of the remuneration policy approved by our general meeting of shareholders from time to time, as required by Dutch law, the remuneration committee will be responsible for, among other things:

- reviewing and making recommendations to the supervisory board with respect to compensation of our management board and supervisory board members;
- reviewing and approving the compensation, including equity compensation, change-of-control benefits and severance arrangements, of our chief executive officer, chief financial officer and such other members of our management as it deems appropriate;
- overseeing the evaluation of our management;
- reviewing periodically and making recommendations to our supervisory board with respect to any incentive compensation and equity plans, programs or similar arrangements;
- exercising the rights of our supervisory board under any equity plans, except for the right to amend any such plans unless otherwise expressly authorized to do so; and
- attending to such other matters as are specifically delegated to our compensation committee by our supervisory board from time to time.

#### ***Nominating and Corporate Governance Committee***

Upon the completion of this offering, our nominating and corporate governance committee will consist of Messrs. \_\_\_\_\_, \_\_\_\_\_ and \_\_\_\_\_. Each member satisfies the independence requirements of the NASDAQ listing standards. The nominating and corporate governance committee will assist the supervisory board in selecting individuals qualified to become our supervisory directors and in determining the composition of the supervisory board and its committees. The nominating and corporate governance committee will be responsible for, among other things:

- recommending to the supervisory board persons to be nominated for election or re-election to the supervisory board at any meeting of the shareholders;
- overseeing the supervisory board's annual review of its own performance and the performance of its committees; and
- considering, preparing and recommending to the supervisory board a set of corporate governance guidelines.

#### **Other Corporate Governance Matters**

The Sarbanes-Oxley Act of 2002, as well as related rules subsequently implemented by the SEC, requires foreign private issuers, including our company, to comply with various corporate governance practices. In addition, NASDAQ rules provide that foreign private issuers may follow home country practice in lieu of the NASDAQ corporate governance standards, subject to certain exceptions and except to the extent that such exemptions would be contrary to U.S. federal securities laws. The home country practices followed by our company in lieu of NASDAQ rules are described below:

- We do not intend to follow NASDAQ's quorum requirements applicable to meetings of shareholders. In accordance with Dutch law and generally accepted business practice, our articles of association do not provide quorum requirements generally applicable to general meetings of shareholders.
- We do not intend to follow NASDAQ's requirements regarding the provision of proxy statements for general meetings of shareholders. Dutch law does not have a regulatory regime for the solicitation of proxies and the solicitation of proxies is not a generally accepted business practice in the Netherlands. We do intend to provide shareholders with an agenda and other relevant documents for the general meeting of shareholders.

We intend to take all actions necessary for us to maintain compliance as a foreign private issuer under the applicable corporate governance requirements of the Sarbanes-Oxley Act of 2002, the rules adopted by the SEC and NASDAQ's listing standards. As a Dutch company listed on a government-recognized stock



exchange, we are required to apply the provisions of the DCGC, or explain any deviation from the provisions of such code in our Dutch annual report required by Dutch law.

Because we are a foreign private issuer, our supervisory board members, management board members and senior management are not subject to short-swing profit and insider trading reporting obligations under section 16 of the U.S. Securities Exchange Act of 1934, as amended (the "Exchange Act"). They will, however, be subject to the obligations to report changes in share ownership under section 13 of the Exchange Act and related SEC rules.

## Compensation

### Supervisory Board Compensation

The members of our supervisory board receive board fees of €20,000 per year. The chairman receives board fees of €30,000 per year. In addition, members receive €1,500 for attendance in person at each supervisory board meeting and €500 for attendance by telephone. In 2012, the aggregate compensation paid to our supervisory directors was €255,000, consisting of the payments set forth below:

(€ in thousands)	SHARE-BASED PAYMENTS <sup>(1)</sup>	BOARD FEE	2012 TOTAL
Ferdinand Verdonck	14	29	43
Sander van Deventer <sup>(2)</sup>	—	8	8
Joseph Feczko	40	29	69
Francois Meyer	40	29	69
Sander Slootweg <sup>(3)</sup>	—	—	—
Philippe Van Holle <sup>(4)</sup>	40	26	66
<b>Total</b>	<b>134</b>	<b>121</b>	<b>255</b>

<sup>(1)</sup> The share-based payment reflects the value of share options granted during the year, as required by IFRS.

<sup>(2)</sup> Following the combination of uniQure and AMT on April 5, 2012, Professor van Deventer has received no remuneration.

<sup>(3)</sup> Appointed April 5, 2012; Mr. Slootweg receives no remuneration.

<sup>(4)</sup> Resigned January 1, 2013.

### Management Board and Other Senior Management Compensation

The table below sets out a breakdown of the compensation in 2012 of the members of the management board and senior management:

(€ in thousands)	SHORT TERM EMPLOYEE BENEFITS	SHARE-BASED PAYMENTS <sup>(1)</sup>	POST- EMPLOYMENT BENEFITS	OTHER LONG TERM BENEFITS	TERMINATION BENEFITS	TOTAL
Jörn Aldag	437	359	64	—	—	860
Piers Morgan	258	150	28	—	—	436
<b>Total for Management Directors</b>	<b>695</b>	<b>509</b>	<b>92</b>	<b>—</b>	<b>—</b>	<b>1,296</b>
Senior Management	689	452	41	—	—	1,182
<b>Total</b>	<b>1,384</b>	<b>961</b>	<b>133</b>	<b>—</b>	<b>—</b>	<b>2,478</b>

<sup>(1)</sup> The share-based payment reflects the value of options granted during the year together with a charge for the period to April 5, 2012 in respect of options granted by AMT.

Our Chief Executive Officer, Jörn Aldag, is entitled to a bonus in the event of a sale of our company equal to 1% of the total consideration payable in such sale. Such bonus would take the form of consideration received by our shareholders in connection with such sale, and would be payable as and when the consideration is paid to our shareholders.

### **2013 Share Incentive Plan**

Our 2013 Plan was adopted by our supervisory board and approved by our shareholders in \_\_\_\_\_, 2013. We will begin making grants under the 2013 Plan following the effective date of the Registration Statement of which this prospectus forms a part. The 2013 Plan provides for the grant of incentive share options, non-statutory share options, share appreciation rights, restricted share awards, restricted share units and other share-based or cash awards. Upon effectiveness of the plan, the number of shares that will be reserved for issuance under the 2013 Plan will be \_\_\_\_\_. Our employees, officers, directors, consultants and advisors are eligible to receive awards under the 2013 Plan. However, incentive share options may only be granted to our employees. The maximum number of ordinary shares with respect to which awards may be granted to any participant under the 2013 Plan is 1,000,000 per calendar year. For purposes of this limit on the maximum number of shares that may be awarded to any participant, the combination of an option in tandem with a share appreciation right will be treated as a single award.

Pursuant to the terms of the 2013 Plan, our supervisory board administers the plan and, subject to any limitations in the plan, selects the recipients of awards and determines:

- the number of ordinary shares covered by options and the dates upon which the options become exercisable;
- the type of options to be granted;
- the duration of options, which may not be in excess of ten years;
- the exercise price of options, which must be at least equal to the fair market value of our ordinary shares on the date of grant; and
- the number of ordinary shares subject to and the terms of any share appreciation rights, restricted share awards, restricted share units or other share-based awards and the terms and conditions of such awards, including conditions for repurchase, issue price and repurchase price.

If our supervisory board delegates authority to an executive officer to grant awards under our 2013 Plan, the executive officer will have the power to make awards to all of our employees, except herself or himself, any other executive officer and any other person that our supervisory board may from time to time designate in writing as not being eligible. Our supervisory board will fix the terms of the awards to be granted by such executive officer, including the exercise price of such awards, and the maximum number of shares subject to awards that such executive officer may make.

Upon a merger or other reorganization event, our supervisory board may, in its sole discretion, take any one or more of the following actions pursuant to the 2013 Plan as to some or all outstanding awards other than restricted shares:

- provide that all outstanding awards shall be assumed, or substantially equivalent awards shall be substituted, by the acquiring or successor corporation (or an affiliate thereof);
- upon written notice to a participant, provide that all of the participant's unexercised awards will terminate immediately prior to the consummation of such reorganization event unless exercised by the participant;
- provide that outstanding awards shall become exercisable, realizable or deliverable, or restrictions applicable to an award shall lapse, in whole or in part, prior to or upon such reorganization event;
- in the event of a reorganization event pursuant to which holders of ordinary shares will receive a cash payment for each share surrendered in the reorganization event, make or provide for a cash payment to the participants with respect to each award held by a participant equal to (1) the number of

ordinary shares subject to the vested portion of the award (after giving effect to any acceleration of vesting that occurs upon or immediately prior to such reorganization event) multiplied by (2) the excess, if any, of the cash payment for each share surrendered in the reorganization event over the exercise, measurement or purchase price of such award and any applicable tax withholdings, in exchange for the termination of such award;

- provide that, in connection with a liquidation or dissolution, awards shall convert into the right to receive liquidation proceeds.

Our supervisory board does not need to take the same action with respect to all awards and may take different actions with respect to portions of the same award.

In the case of certain restricted share units, no assumption or substitution is permitted, and the restricted share units will instead be settled in accordance with the terms of the applicable restricted share unit agreement.

Upon the occurrence of a reorganization event other than a liquidation or dissolution, the repurchase and other rights with respect to outstanding restricted share awards will continue for the benefit of the successor company and will, unless the supervisory board may otherwise determine, apply to the cash, securities or other property into which our ordinary shares are converted or exchanged pursuant to the reorganization event. Upon the occurrence of a reorganization event involving a liquidation or dissolution, all restrictions and conditions on each outstanding restricted share award will automatically be deemed terminated or satisfied, unless otherwise provided in the agreement evidencing the restricted share award.

At any time, our supervisory board may, in its sole discretion, provide that any award under the 2013 Plan will become immediately exercisable in full or in part, free of some or all restrictions or conditions, or otherwise realizable in full or in part.

No award may be granted under the 2013 Plan on or after \_\_\_\_\_, 2023. Our supervisory board may amend, suspend or terminate the 2013 Plan at any time, except that shareholder approval may be required to comply with applicable law or stock market requirements. While our ordinary shares are listed on the NASDAQ Global Market, we may not, without shareholder consent, amend, cancel or take any action under the 2013 Plan that constitutes a "repricing" within the meaning of the rules of the NASDAQ Global Market.

### **2012 Option Plan**

We adopted our 2012 Option Plan in June 2012, which we refer to as the 2012 plan. As of September 30, 2013, a total of 10,906,414 shares have been approved to be issued pursuant to options granted under the 2012 Option Plan.

The 2012 plan allows us to grant options to acquire ordinary shares to employees of uniQure and its subsidiaries as determined from time to time by our management board after authorization from our supervisory board. The 2012 plan is administered and all grants under the 2012 plan must be approved by our supervisory board. All grants of options to members of our management board and supervisory board must also be approved by the general meeting of shareholders to the extent required by Dutch law.

Options granted under the 2012 plan generally vest at a rate of one-third on the first anniversary of the grant date with the remainder vesting on a straight line daily basis over the next two years.

Substantially all options under our 2012 plan will vest in full upon the completion of this offering. No further grants will be made under the 2012 plan following the closing of this offering.

## RELATED-PARTY TRANSACTIONS

Since January 1, 2010, we have engaged in the following transactions with the members of our supervisory board, management board, senior management, holders of class A ordinary shares, and their affiliates, which we refer to as our related parties.

### 2012 and 2013 Convertible Notes

In December 2012, January 2013 and March 2013, we sold convertible promissory notes in the aggregate principal amount of €13.5 million in a private placement to certain of our existing investors, which we refer to as the convertible notes. The convertible notes accrued interest at a rate equal to 8% per year, and had a maturity date of December 31, 2014, unless previously converted. No payments of principal or interest were made under these notes. In addition, in connection with the issuance of the convertible notes we issued the holders of such convertible notes warrants to purchase an aggregate of 668,163 of our class A ordinary shares. In July 2013, the convertible notes were converted into an aggregate of 6,681,678 of our class A ordinary shares.

The following table sets forth the participation in this financing by our related parties:

PURCHASER	AGGREGATE PRINCIPAL AMOUNT OF CONVERTIBLE NOTES	CLASS A ORDINARY SHARES ISSUED UPON CONVERSION OF CONVERTIBLE NOTES	CLASS A ORDINARY SHARES ISSUABLE UPON EXERCISE OF WARRANTS
Forbion Co-Investment Cooperatief U.A. (1)	€ 1,000,000	495,049	49,504
Cooperatieve Gilde Healthcare II U.A.	€ 1,000,000	495,049	49,504
Collier International Partners V-A, L.P.	€10,000,000	4,950,495	495,049
Lupus Alpha	€ 1,000,000	495,049	49,504
Grupo Netco	€ 497,000	246,036	24,602

<sup>(1)</sup> Sander Slootweg, a member of our supervisory board, is an Managing Partner at Forbion Capital Partners. Sander van Deventer, a member of our supervisory board, is a partner at Forbion Capital Partners.

### 2012 Share Purchase Incentive Plan

In November 2012, we raised an aggregate of €552,202 through the issue of class B ordinary shares at a price of €0.614 per share in part to members of our supervisory board and senior management, including Joseph Feczko, Francois Meyer, Ferdinand Verdonck, Piers Morgan and Hans Christian Rohde.

### 2012 Public to Private Transaction

In April 2012, we completed the acquisition of the business of AMT. In connection with this transaction, we issued 31,101,065 ordinary shares to the AMT shareholders as consideration for the business of AMT.

The following table sets forth the number of ordinary shares received by our related parties.

SHAREHOLDER	NUMBER OF ORDINARY SHARES
Entities affiliated with Forbion <sup>(1)</sup>	5,987,685
Cooperatieve Gilde Healthcare II U.A.	6,081,803
Ferdinand Verdonck	131,178
Sander van Deventer	49,298
Joseph M. Feczko	118,843
François Meyer	88,860
Jörn Aldag	119,299
Piers Morgan	21,765
Harald Petry	3,137
Hans Preusting	12,639

#### Issuance of Class A Ordinary Shares Pursuant to Conversion of Convertible Notes

As part of the transaction with AMT, we assumed a €5.0 million convertible loan provided to AMT by Forbion in December 2009. On April 5, 2012, this loan, together with accrued interest of €320,000, was converted into our class A ordinary shares at a price of €1.00 per share, resulting in the issue to Forbion of 5,320,000 class A ordinary shares. The terms of the conversion represented an amendment to the original conversion price of €3.69 per AMT share. This amendment to the conversion terms formed part of the terms of the acquisition.

#### Issuance of Class A Ordinary Shares to Forbion

On April 5, 2012, we raised €6.0 million through an issue to Forbion of 9,771,987 of our class A ordinary shares at a price of €0.614 per share.

#### Issuance of Class A Ordinary Shares to Gilde

On May 18, 2012, we raised a further €1.0 million through the issue of 1,628,664 of our class A ordinary shares to Gilde at a price of €0.614 per share.

#### Issuance of AMT Ordinary Shares

In October 2010, AMT issued 8.4 million of its ordinary shares at a price per share of €1.70 for a purchase price of €14.3 million.

The following table sets forth the participation in this financing by our executive officers, entities affiliated with our directors and our ten percent shareholders and their affiliates.

PURCHASER	AMT ORDINARY SHARES PURCHASED
Forbion Capital Fund I Cooperatief U.A. <sup>(1)</sup>	588,235
Cooperatieve Gilde Healthcare II U.A.	882,353
Jörn Aldag	29,412
Piers Morgan	11,765

<sup>(1)</sup> Sander Slootweg, a member of our supervisory board, is a managing partner at Forbion Capital Partners. Sander van Deventer, a member of our supervisory board, is a partner at Forbion Capital Partners.

## **Advisory Agreement**

Dr. Van Deventer, who served as our interim Chief Executive Officer from February to November 2010 and currently serves as an advisor to us and as a member of our supervisory board, is a partner of Forbion. In 2010, 2011 and 2012, Dr. Van Deventer received advisory fees of €19,000, €56,000 and €8,000 from us.

## **Shareholders Agreements**

### ***Class A Shareholders Agreement***

On April 19, 2012, we, entities affiliated with Forbion and Cooperatieve Gilde Healthcare II U.A, entered into a class A shareholders agreement, which we refer to as the class A shareholders agreement.

The shareholders agreement includes provisions related to:

- registration rights in respect of our shares, in case they are listed on a United States securities exchange, which rights have been waived in connection with this offering
- the management of our company, including nomination rights relating to our supervisory board and our management board, consent rights of our supervisory board, approval rights of shareholders, pre-emptive rights, dividends and voting; and
- the transfer of shares, including the prohibition of share transfers unless permitted by the shareholders

Upon consummation of this offering, the class A shareholders agreement will terminate.

### ***Class B Shareholders Agreement***

On April 19, 2012, we, entities affiliated with Forbion, Cooperatieve Gilde Healthcare II U.A and Stichting Administratiekantoor uniQure, B.V., entered into a class B shareholders agreement, which we refer to as the class B shareholders agreement.

The shareholders agreement includes provisions related to:

- the management of our company, including nomination rights relating to our supervisory board and our management board, consent rights of our supervisory board, approval rights of shareholders, pre-emptive rights, dividends and voting; and
- the transfer of shares, including the prohibition of share transfers unless permitted by the shareholders

Upon consummation of this offering, the class B shareholders agreement will terminate.

### ***Class C Shareholders Agreement***

On July 8, 2013, we, our existing shareholders, Stichting Administratiekantoor uniQure, B.V. and Chiesi Farmaceutici S.p.A., entered into a class C shareholders agreement, which we refer to as the class C shareholders agreement.

The shareholders agreement includes provisions related to:

- pre-emptive rights and dividends; and
- the transfer of shares, including the prohibition of share transfers unless permitted by the shareholders

Upon consummation of this offering, the class C shareholders agreement will terminate.

## PRINCIPAL SHAREHOLDERS

The following table sets forth information with respect to the beneficial ownership of our ordinary shares as of September 30, 2013 by:

- each of the members of our management board and supervisory board;
- each of our other members of senior management; and
- each person, or group of affiliated persons, who is known by us to beneficially own more than 5% of our ordinary shares.

The column entitled "Percentage of Shares Beneficially Owned—Before Offering" is based on a total of 60,948,979 ordinary shares outstanding as of September 30, 2013, and gives effect to the conversion of our class A, class B and class C ordinary shares into ordinary shares prior to the closing of this offering.

The column entitled "Percentage of Shares Beneficially Owned—After Offering" also gives effect to \_\_\_\_\_ ordinary shares that we are selling in this offering, but not including any additional shares issuable upon exercise of outstanding options.

Beneficial ownership is determined in accordance with the rules and regulations of the SEC and includes voting or investment power with respect to our ordinary shares. Ordinary shares subject to options that are currently exercisable or exercisable within 60 days of September 30, 2013 are considered outstanding and beneficially owned by the person holding the options for the purpose of calculating the percentage ownership of that person but not for the purpose of calculating the percentage ownership of any other person. Except as otherwise noted, the persons and entities in this table have sole voting and investing power with respect to all of our ordinary shares beneficially owned by them, subject to community property laws, where applicable. Except as otherwise set forth below, the address of the beneficial owner is c/o uniQure B.V., Meibergdreef 61, 1105 BA, Amsterdam, the Netherlands.

NAME AND ADDRESS OF BENEFICIAL OWNER	NUMBER OF SHARES BENEFICIALLY OWNED	PERCENTAGE OF SHARES BENEFICIALLY OWNED	
		BEFORE OFFERING	AFTER OFFERING
<b>5% Shareholders:</b>			
Entities affiliated with Forbion <sup>(1)</sup>	21,673,522	35.6%	
Coöperatieve Gilde Healthcare II U.A. <sup>(2)</sup>	8,255,020	13.5%	
Entities affiliated with Advent <sup>(3)</sup>	3,761,612	6.2%	
Coller International Partners V-A, L.P. <sup>(4)</sup>	27,119,066	44.5%	
Chiesi Farmaceutici S.p.A. <sup>(5)</sup>	5,546,070	9.1%	
<b>Management Board Members, Supervisory Board Members and Senior Management</b>			
Ferdinand Verdonck <sup>(6)</sup>	609,542	1%	
Sander Slootweg <sup>(7)</sup>	21,673,522	35.6%	
Sander van Deventer <sup>(8)</sup>	21,673,522	35.6%	
Joseph M. Feczko <sup>(9)</sup>	242,116	*	
François Meyer <sup>(10)</sup>	192,133	*	
Paula Soteropoulos	—	—	
Jörn Aldag <sup>(11)</sup>	1,048,758	1.7%	
Piers Morgan <sup>(12)</sup>	526,303	*	
Philip Astley-Sparke <sup>(13)</sup>	21,673,522	35.6%	
Christian Meyer	—	—	
Harald Petry <sup>(14)</sup>	390,411	*	
Hans Preusting <sup>(15)</sup>	399,913	*	
Hans Christian Rohde	65,500	*	

\* Represents beneficial ownership of less than one percent of our outstanding ordinary shares.

(1) Consists of (i) 4,938,367 ordinary shares held by Coöperatieve AAC LS U.A., or Coöperatieve; (ii) 7,308,884 ordinary shares held by Forbion Co-Investment Coöperatief U.A., or FCI; (iii) 9,327,469 ordinary shares held by Forbion Co-Investment II Coöperatief U.A., or FCI II; (iv) warrants held by FCI to purchase 49,504 ordinary shares that are exercisable as of September 30, 2013 or will become exercisable within 60 days after such date; and (v) 49,298 ordinary shares held by SJH van Deventer CV, or SJH. Forbion 1 Management B.V., the director of Coöperatieve and FCI, and Forbion 1 Co II Management B.V., the director of FCI II, and Forbion Capital Partners Management Services B.V., or Forbion Capital Partners, the general partner of SJH, may be deemed to have voting and dispositive power over the ordinary shares held by Coöperatieve, FCI, FCI II and SJH. Investment decisions with respect to the ordinary shares held by Coöperatieve, FCI, FCI II and SJH can be made by any two of the duly authorized representatives of Coöperatieve, FCI, FCI II and SJH. Mr. Slootweg and Dr. van Deventer are partners of Forbion Capital Partners, which acts as the investment advisor to the directors of Coöperatieve, FCI, FCI II and as General Partner to SJH. Mr. Astley-Sparke, among others, as a venture partner acts as an independent contractor in an advisory function to Forbion Capital Partners. Each of Mr. Slootweg, Dr. van Deventer and Mr. Astley-Sparke disclaim beneficial ownership of such ordinary shares, except to the extent of his pecuniary interest therein. The address of Forbion Capital Partners, Coöperatieve, FCI, FCI II and SJH is Gooimeer 2-35, 1411 DC Naarden, The Netherlands.

(2) Consists of (i) 8,205,516 ordinary shares held by Coöperatieve Gilde Healthcare II U.A. and (ii) warrants held by Coöperatieve Gilde Healthcare II U.A. to purchase 49,504 ordinary shares that are exercisable as of September 30, 2013 or will become exercisable within 60 days after such date. The manager of Coöperatieve Gilde Healthcare II U.A. is Gilde Healthcare II Management B.V., or Gilde Management, and Gilde Management is owned by Gilde Healthcare Holding B.V., or Gilde Holding. Three managing partners, Edwin de Graaf, Marc Olivier Perret and Martenmanshurk B.V. (of which Pieter van der Meer is the owner and manager) each own 28.66% of Gilde Holding and Stichting Administratiekantoor Gilde Healthcare Holding, or Stichting, owns 14% of Gilde Holding. Stichting is controlled by Mr. de Graaf, Mr. Perret and Martenmanshurk B.V. and issued depository receipts for shares in Gilde Holding to two partners, Arthur Franken and Dirk Kersten. Each of Mr. de Graaf, Mr. Perret and Mr. van der Meer share voting and dispositive power of the shares, and



disclaim beneficial ownership of the shares except to the extent of their respective pecuniary interest therein. The address of Coöperatieve Gilde Healthcare II U.A. is Newtonlaan 91, 3584 BP, Utrecht, The Netherlands.

- (3) Consists of (i) 3,724,371 ordinary shares beneficially owned by Advent Private Equity Fund IV LP and (ii) 37,241 ordinary shares held by Advent Management IV LP. Advent Venture Partners LLP is the manager of the Advent funds and may be deemed to have voting and dispositive power over the ordinary shares held by them. The registered office of Advent Management IV LP is 50 Lothian Road, Festival Square, Edinburgh, EH3 9WJ, United Kingdom.
- (4) Consists of (i) 4,950,495 ordinary shares held by Collier International Partners V-A, L.P., or Collier; (ii) warrants held by Collier to purchase 495,049 ordinary shares that are exercisable as of September 30, 2013 or will become exercisable within 60 days after such date; (iii) 2,889,438 ordinary shares held by Coöperatieve; (iv) 5,098,677 ordinary shares held by FCI; (v) 6,529,228 ordinary shares held by FCI II; and (vi) warrants held by FCI to purchase 34,533 shares that are exercisable as of September 30, 2013 or will become exercisable within 60 days after such date. Collier is a limited partner of the Forbion funds. Collier has no dispositive or voting power over ordinary shares held by the Forbion funds and disclaims beneficial ownership of such ordinary shares except to the extent of its pecuniary interest therein. See Note 1. The general partner of Collier is Collier International General Partner V, L.P. of which Collier Investment Management Limited, or CIML, is the general partner. The directors of CIML are Jeremy Joseph Collier, Cyril Joseph Mahon, Roger Alan Le Tissier, Paul McDonald, Peter Michael Hutton, John Charlton Loveless and Andrew Thane Maden Hitchon and may be deemed to share voting and dispositive power with respect to the ordinary shares held by Collier. The CIML directors disclaim beneficial ownership of such ordinary shares except to the extent of their pecuniary interest therein. The address of Collier is c/o Collier Investment Management Limited, PO Box 255, Trafalgar Court, Les Banques, St Peter Port, Guernsey, Channel Islands.
- (5) The registered office of Chiesi Farmaceutici S.p.A is Via Palermo, 26, 43122 Parma, Italy.
- (6) Consists of 377,178 ordinary shares and options to purchase 232,364 ordinary shares that are exercisable as of September 30, 2013 or will become exercisable within 60 days after such date.
- (7) Consists of securities held by entities affiliated with Forbion. See Note 1.
- (8) Consists of (i) securities held by funds affiliated with Forbion and (ii) 49,298 ordinary shares held by SJH. See Note 1. Dr. van Deventer is the sole shareholder of SJH. Dr. van Deventer disclaims beneficial ownership of such shares, except to the extent of his pecuniary interest therein.
- (9) Consists of 138,843 ordinary shares and options to purchase 103,273 ordinary shares that are exercisable as of September 30, 2013 or will become exercisable within 60 days after such date.
- (10) Consists of 88,860 ordinary shares and options to purchase 103,273 ordinary shares that are exercisable as of September 30, 2013 or will become exercisable within 60 days after such date.
- (11) Consists of 119,299 ordinary shares and options to purchase 929,459 ordinary shares that are exercisable as of September 30, 2013 or will become exercisable within 60 days after such date.
- (12) Consists of 139,029 ordinary shares and options to purchase 387,274 ordinary shares that are exercisable as of September 30, 2013 or will become exercisable within 60 days after such date.
- (13) Consists of securities held by entities affiliated with Forbion. See Note 1.
- (14) Consists of 3,137 ordinary shares and options to purchase 387,274 ordinary shares that are exercisable as of September 30, 2013 or will become exercisable within 60 days after such date.
- (15) Consists of 12,639 ordinary shares and options to purchase 387,274 ordinary shares that are exercisable as of September 30, 2013 or will become exercisable within 60 days after such date.

## Holdings by U.S. Shareholders

As of September 30, 2013, there were no holders of record of ordinary shares located in the United States.

## DESCRIPTION OF SHARE CAPITAL

### General

We were incorporated on January 9, 2012 as a private company with limited liability (*besloten vennootschap met beperkte aansprakelijkheid*) under Dutch law. On or prior to completion of this offering, we intend to convert into a public company with limited liability (*naamloze vennootschap*) pursuant to a deed of amendment and conversion, which we refer to as the Deed of Amendment and Conversion, and our legal name will be UniQure N.V.

Our company is registered with the Dutch Trade Register of the Chamber of Commerce (*handelsregister van de Kamer van Koophandel en Fabrieken*) in Amsterdam, the Netherlands under number 54385229. Our corporate seat is in Amsterdam, the Netherlands, and our registered office is at Meibergdreef 61, 1105 BA Amsterdam, the Netherlands.

As of the date of this prospectus, our share capital is divided into class A, B and C ordinary shares. All of our outstanding shares will be converted into ordinary shares pursuant to the Deed of Amendment and Conversion on or prior to completion of this offering. Our authorized share capital at the date of this prospectus amounts to €2,000,000. Our issued share capital at the date of this prospectus amounts to .

As of the execution of the Deed of Amendment and Conversion, our authorized share capital will be , divided into ordinary shares, each with a nominal value of €0.05, and preference shares, each with a nominal value of €0.05. Under Dutch law, our authorized share capital is the maximum capital that we may issue without amending our articles of association.

We have adopted an anti-takeover measure pursuant to which our management board may, subject to supervisory board approval but without approval of the general meeting of shareholders, issue (or grant the right to acquire) preference shares. We may issue an amount of preference shares up to an amount equal to 100% of our issued share capital as per the moment immediately prior to the issuance of such preferred shares. The preference shares will then be issued to a separate, newly established foundation. The purpose clause in this foundation's articles of association will provide that it will act to serve the best interests of us, our associated business and all parties connected to us, by opposing any influences that conflict with these interests and threaten to undermine our continuity, independence and identity. This foundation will be structured to operate independently from us.

The preference shares will be issued to the foundation for their nominal value, of which only 25% will be due upon issuance. The voting rights of our shares are based on nominal value and, as we expect our shares to trade substantially in excess of nominal value, preference shares issued at nominal value will obtain significant voting power for a substantially reduced price and thus be used as a defensive measure. These preference shares will have both a liquidation and dividend preference over our ordinary shares and will accrue cash dividends at a fixed rate.

Our management board may issue these preference shares to protect us from influences that do not serve our best interests and threaten to undermine our continuity, independence and identity. These influences may include a third-party acquiring a significant percentage of our ordinary shares, the announcement of a public offer for our ordinary shares, other concentration of control over our ordinary shares or any other form of pressure on us to alter our strategic policies.

We intend to apply for the listing of our ordinary shares on the Nasdaq Global Market under the symbol "QURE".

Initial settlement of the ordinary shares offered in this offering is expected to take place on or about the completion date of this offering through The Depository Trust Company, or DTC, in accordance with its

customary settlement procedures for equity securities. Each person owning ordinary shares held through DTC must rely on the procedures thereof and on institutions that have accounts therewith to exercise any rights of a holder of the ordinary shares.

## Articles of Association and Dutch law

We refer to our articles of association as of the date of this prospectus as our "current articles." When we refer to our "articles of association" in this prospectus, we mean our articles of association as they will be in force after the execution of the Deed of Amendment and Conversion which is expected to take place prior to the consummation of this offering.

Our current articles were last amended by a deed of amendment, executed on July 24, 2013. We intend to further amend our current articles and convert our company into a public company with limited liability (*naamloze vennootschap*) effective prior to the consummation of this offering. On , 2014 the general meeting of shareholders, resolved to amend the current articles and to convert into a public company with limited liability by means of the Deed of Amendment and Conversion, subject to completion of this offering. The draft Deed of Amendment and Conversion has been made available to the shareholders in advance of the date of the resolution and remains available for inspection by interested parties at our offices in Amsterdam, the Netherlands up to and including the completion of this offering.

Set forth below is a summary of relevant information concerning the material provisions of our articles of association and applicable Dutch law. This summary does not constitute legal advice regarding those matters and should not be regarded as such.

## Amendment of Articles of Association

The general meeting of shareholders, at the proposal of our management board, with the prior approval of our supervisory board, may resolve to amend our articles of association. A resolution taken by the general meeting of shareholders to amend our articles of association requires a simple majority of the votes cast.

## Company's Shareholder Register

Subject to Dutch law and the articles of association, we must keep our shareholders' register accurate and up-to-date. Our management board keeps our shareholders' register and records names and addresses of all holders of shares, showing the date on which the shares were acquired, the date of the acknowledgement by or notification of us as well as the amount paid on each share. The shareholders' register also includes the names and addresses of those with a right of use and enjoyment (*vruchtgebruik*) in shares belonging to another or a pledge over shares. The ordinary shares offered in this offering will be held through DTC, therefore DTC will be recorded in the shareholders register as the holder of the ordinary shares.

## Corporate objectives

Under Article 3 of the current articles of association, our corporate objectives are:

- to research, develop, produce and commercialize products, services and technology in the biopharmaceutical sphere;
- to incorporate, participate in, conduct the management of and take any other financial interest in other companies and enterprises;
- to render administrative, technical, financial, economic or managerial services to other companies, persons or enterprises;
- to acquire, dispose of manage and exploit real and personal property, including patents, marks, licenses, permits and other intellectual property rights;
- to borrow and/or lend moneys, act as surety or guarantor in any other manner, and bind itself jointly and severally or otherwise in addition to or on behalf of others,

the foregoing, whether or not in collaboration with third parties, and inclusive of the performance and promotion of all activities which directly and indirectly relate to those objects, all this in the broadest sense

### **Limitation on liability and indemnification matters**

Under Dutch law, managing directors, supervisory directors and certain other representatives may be held liable for damages in the event of improper or negligent performance of their duties. They may be held jointly and severally liable for damages to the company and to third parties for infringement of the articles of association or of certain provisions of the Dutch Civil Code. In certain circumstances, they may also incur additional specific civil and criminal liabilities. We have a policy insuring managing directors, supervisory directors and certain other representatives against damages resulting from their conduct when acting in their capacities as such directors or representatives. In addition, our articles of association provide for indemnification of our managing directors and supervisory directors, including reimbursement for reasonable legal fees and damages or fines incurred based on acts or failures to act in the performance of their duties. Such indemnification will not be available in instances of willful (*opzettelijk*), intentionally reckless (*bewust roekeloos*) or seriously culpable (*ernstig verwijtbaar*) conduct unless Dutch law provides otherwise.

### **Shareholders' meetings and consents**

#### *General meeting*

General meetings of shareholders are held in Amsterdam, or in the municipality of Haarlemmermeer (Schiphol Airport), the Netherlands. The annual general meeting of shareholders must be held within six months of the end of each financial year. Additional extraordinary general meetings of shareholders may also be held whenever considered appropriate by the management board or the supervisory board.

Pursuant to Dutch law, one or more shareholders, who alone or jointly represent at least one-tenth of the issued capital, may, on their application, be authorized by the Dutch court to convene a general meeting of shareholders. The Dutch court will disallow the application if it does not appear that the applicants have previously requested that the management board and the supervisory board convene a general meeting of shareholders and neither the management nor the supervisory board has taken the necessary steps so that the general meeting of shareholders could be held within six weeks after the request.

General meetings of shareholders are convened by a notice which includes an agenda stating the items to be discussed. For the annual general meeting of shareholders the agenda will include, among other things, the adoption of our annual accounts, the appropriation of our profits and proposals relating to the composition of the management board and/or the supervisory board, including filling any vacancies in the management board and/or the supervisory board. In addition, the agenda for the general meeting of shareholders includes such items as have been (1) included therein by the management board or the supervisory board and (2) requested by one or more shareholders and/or others entitled to attend general meetings of shareholders representing at least 3% of the issued share capital of a company or such lower percentage as the articles of association may provide. Our articles of association do not state such lower percentage. Such requests must be made in writing and received by the management board at least sixty days before the day of the meeting. Our management board may decide not to place items so requested on the agenda, if it believes that doing so would be detrimental to our vital interests. No resolutions will be adopted on items other than those which have been included in the agenda.

Pursuant to our articles of association, the general meeting of shareholders is chaired by the chairman of the supervisory board. However, the chairman may charge another person to chair the general meeting in his place even if he is present at the meeting. If the chairman of our supervisory board is absent and has not charged another person to chair the meeting in his place, the supervisory directors present at the meeting shall appoint one of them to be chairman. If no supervisory directors are present at the general meeting of shareholders, the general meeting of shareholders will be chaired by the chairman of our management board or, if the chairman of our management board is absent, by one of the other managing directors designated

for that purpose by the management board. Managing directors and supervisory directors may attend a general meeting of shareholders. In these meetings, they have an advisory vote. The chairman of the meeting may decide at his discretion to admit other persons to the meeting.

All shareholders and others entitled to attend general meetings of shareholders are authorized to attend the general meeting of shareholders, to address the meeting and, in so far as they have such right, to vote.

#### *Quorum and voting requirements*

Each ordinary share confers the right on the holder thereof to cast one vote at the general meeting of shareholders. Shareholders may vote by proxy. The voting rights attached to any shares held by us are suspended as long as they are held in treasury. If a right of use and enjoyment (*vruchtgebruik*) or a right of pledge over ordinary shares was granted prior to the time such ordinary share was acquired by us, the holders of such right of use and enjoyment in ordinary shares belonging to another and the holders of a right of pledge in respect of ordinary shares held by us are not excluded from any right such holders may have to vote on such ordinary shares. We may not cast votes in respect of a share in respect of which there is a right of use and enjoyment or a right of pledge. Shares which are not entitled to voting rights pursuant to the preceding sentences will not be taken into account for the purpose of determining the number of shareholders that vote and that are present or represented, or the amount of the share capital that is present or that is represented, at a general meeting of shareholders.

In accordance with Dutch law and generally accepted business practices, our articles of association do not provide quorum requirements generally applicable to general meetings of shareholders. To this extent, our practice varies from the requirement of Nasdaq Listing Rule 5620(c), which requires an issuer to provide in its bylaws for a generally applicable quorum, and that such quorum may not be less than one-third of the outstanding voting shares.

Decisions are made at the general meeting of shareholders by an absolute majority of votes cast, except where Dutch law or our articles of association provide for a qualified majority or unanimity.

#### ***Managing directors and supervisory directors***

##### *Election of managing directors and supervisory directors*

Under our articles of association, the managing directors and supervisory directors are appointed by the general meeting of shareholders, upon nomination by our supervisory board. However, the shareholders at the general meeting of shareholders may at all times overrule the binding nomination by a resolution adopted by at least a two-thirds majority of the votes cast, provided such majority represents more than half of the issued share capital of our company. If the general meeting of shareholders overrules the binding nomination, the supervisory board shall make a new nomination.

##### *Duties and liabilities of managing directors and supervisory directors*

Under Dutch law, the management board is responsible for our day-to-day management, strategy, policy and operations. The supervisory board is responsible for supervising the conduct of, and providing advice to the management board and for, supervising our business generally. Furthermore, each managing director and supervisory director has a duty to act in the corporate interest of our company. Under Dutch law, the corporate interest extends to the interests of all corporate stakeholders, such as shareholders, creditors, employees, customers and suppliers. The duty to act in the corporate interest of our company also applies in the event of a proposed sale or split-up of our company, whereby the circumstances generally dictate how such duty is to be applied. Any resolution of the management board regarding a significant change in our identity or character requires shareholder approval. For additional information, please see "Shareholder vote on certain reorganizations."

## **Dividends and other distributions**

### *Amount available for distribution*

We may only make distributions to our shareholders if our shareholders' equity exceeds the sum of the paid-up and called-up share capital plus the reserves as required to be maintained by Dutch law or by our articles of association. Under our articles of association, a dividend is first paid out of the profit, if available for distribution, on any preference shares, of which none will be outstanding on completion of this offering. Any amount remaining out of the profit is carried to reserve as the management board determines, subject to the approval of the supervisory board. After reservation by the management board of any profit, the remaining profit will be at the disposal of shareholders. The management board is permitted, subject to certain requirements and subject to approval of the supervisory board, to declare interim dividends without the approval of the general meeting of shareholders. Our corporate policy is that we only make a distribution of dividends to our shareholders after the adoption of our annual accounts demonstrating that such distribution is legally permitted.

Dividends and other distributions shall be made payable not later than the date determined by the management board. Claims to dividends and other distributions not made within five years from the date that such dividends or distributions became payable, will lapse and any such amounts will be considered to have been forfeited to us (*verjaring*).

We do not anticipate paying any cash dividends for the foreseeable future.

### *Exchange controls*

Under Dutch law, there are no exchange controls applicable to the transfer of dividends or other distributions with respect to, or of the proceeds from the sale of, shares in a Dutch company, to persons outside the Netherlands.

### *Squeeze-out proceedings*

Pursuant to Section 92a, Book 2, Dutch Civil Code, a shareholder who for its own account contributes at least 95% of our issued share capital may initiate proceedings against all our minority shareholders jointly for the transfer of their shares to it. The proceedings are held before the Enterprise Chamber of the Court of Appeal in Amsterdam (*Ondernemingskamer*) and can be instituted by means of a writ of summons served upon each of the minority shareholders in accordance with the provisions of the Dutch Code of Civil Procedure (*Wetboek van Burgerlijke Rechtsvordering*). The Enterprise Chamber may grant the claim for squeeze out in relation to all minority shareholders and will determine the price to be paid for the shares, if necessary after appointment of one or three experts who will offer an opinion to the Enterprise Chamber on the value to be paid for the shares of the minority shareholders. Once the order to transfer becomes final before the Enterprise Chamber, the majority shareholder that institutes the squeeze-out proceedings shall give written notice to all minority shareholders whose addresses are known by the majority shareholder of the date and place of payment and the price. Unless the majority shareholder knows the addresses of all minority shareholders, the majority shareholder is required to publish the same in a daily newspaper with a national circulation.

### *Obligation to disclose holdings and transactions*

Pursuant to the Dutch Financial Markets Supervision Act (*Wet op het financieel toezicht*, or FMSA), any managing director or supervisory director and any other person who has managerial or co-managerial responsibilities in respect of us or who has the authority to make decisions affecting our future developments and business prospects and who may have regularly access to inside information relating, directly or indirectly, to us, must give written notice to the Dutch Authority for the Financial Markets, or AFM, by means of a standard form of any transactions conducted for his own account relating to our shares or in financial instruments the value of which is also based on the value of our shares.

Furthermore, in accordance with the FMSA and the regulations promulgated thereunder, certain persons who are closely associated with members of our supervisory board or any of the other persons as described above, are required to notify the AFM of any transactions conducted for their own account relating to our shares or in financial instruments the value of which is also based on the value of our shares. The FMSA and the regulations promulgated thereunder cover the following categories of persons: (1) the spouse or any partner considered by national law as equivalent to the spouse, (2) dependent children of such persons, (3) other relatives who have shared the same household for at least one year at the relevant transaction date, and (4) any legal person, trust or partnership whose, among other things, managerial responsibilities are discharged by a person referred to under (1), (2) or (3) above or by the relevant supervisory director or other person with any authority in respect of us as described above.

The AFM must be notified no later than the fifth business day following the relevant transaction date. Under certain circumstances, notification may be postponed until the date the value of the transactions performed for that person's own account, together with transactions carried out by the persons closely associated with that person, amounts to €5,000 or more in the calendar year in question.

Non-compliance with the notification obligations under the FMSA could lead to criminal fines, administrative fines, imprisonment or other sanctions. In addition, non-compliance with some of the notification obligations under the FMSA may lead to civil sanctions, including suspension of the voting rights relating to our shares held by the offender for a period of not more than three years and a prohibition to own shares or voting rights on our shares for a period of not more than five years.

The AFM does not issue separate public announcements of notifications received by it. It does, however, keep a public register of all notifications under the FMSA on its website, <http://www.afm.nl>. Third parties can request to be notified automatically by e-mail of changes to the public register in relation to a particular company's shares or a particular notifying party.

The FMSA contains rules intended to prevent market abuse, such as insider trading, tipping and market manipulation.

Pursuant to the rules intended to prevent market abuse, prior to the completion of this offering we will adopt an internal code on inside information in respect of the holding of and carrying out of transactions by managing directors, supervisory directors and employees in our shares or in financial instruments the value of which is determined by the value of our shares. Furthermore, we have drawn up a list of those persons working for us who could have access to inside information on a regular or incidental basis and have informed such persons of the rules on insider trading and market manipulation, including the sanctions which can be imposed in the event of a violation of those rules.

### **Comparison of Dutch corporate law and our Articles of Association and Delaware corporate law**

The following comparison between Dutch corporate law, which applies to us, and Delaware corporate law, the law under which many publicly listed companies in the United States are incorporated, discusses additional matters not otherwise described in this prospectus. This summary is subject to Dutch law, including Book 2 of the Dutch Civil Code and Delaware corporation law, including the Delaware General Corporation Law.

### **Corporate governance**

#### *Duties of managing directors and supervisory directors*

*The Netherlands.* We have a two tier board structure consisting of our management board (*raad van bestuur*) and a separate supervisory board (*raad van commissarissen*).

Under Dutch law, the management board is responsible for the day-to-day management and the strategy, policy and operations of a company. The supervisory board is responsible for supervising the conduct of, and

providing advice to, the management board and for supervising the company's general affairs and business. Each managing director and supervisory director has a duty to act in the corporate interest of the company. Under Dutch law, the corporate interest extends to the interests of all corporate stakeholders, such as shareholders, creditors, employees, customers and suppliers. The duty to act in the corporate interest of the company also applies in the event of a proposed sale or split-up of a company, whereby the circumstances generally dictate how such duty is to be applied. Any resolution of the management board regarding a significant change in the identity or character of a company requires shareholders' approval.

*Delaware.* The board of directors bears the ultimate responsibility for managing the business and affairs of a corporation. In discharging this function, directors of a Delaware corporation owe fiduciary duties of care and loyalty to the corporation and to its stockholders. Delaware courts have decided that the directors of a Delaware corporation are required to exercise informed business judgment in the performance of their duties. Informed business judgment means that the directors have informed themselves of all material information reasonably available to them. Delaware courts have also imposed a heightened standard of conduct upon directors of a Delaware corporation who take any action designed to defeat a threatened change in control of the corporation. In addition, under Delaware law, when the board of directors of a Delaware corporation approves the sale or break-up of a corporation, the board of directors may, in certain circumstances, have a duty to obtain the highest value reasonably available to the stockholders.

#### *Supervisory director terms*

*The Netherlands.* Under Dutch law, supervisory directors of a listed company are generally appointed for an individual term of a maximum of four years. A limit of twelve years generally applies. Our supervisory directors are appointed by the general meeting of shareholders for a term of up to four years. A supervisory director may be reappointed for a term of up to four years at a time. A supervisory director may serve on the supervisory board for a period not longer than twelve years, which period may or may not be interrupted, unless resolved otherwise by the general meeting of shareholders.

The general meeting of shareholders, are entitled at all times to suspend or dismiss a supervisory director. The general meeting of shareholders may only adopt a resolution to suspend or dismiss such supervisory director by at least a two-thirds majority of the votes cast, if such majority represents more than half of the issued share capital of the company, unless the proposal was made by the supervisory board, in which case a simple majority of the votes cast is sufficient.

*Delaware.* The Delaware General Corporation Law generally provides for a one-year term for directors, but permits directorships to be divided into up to three classes with up to three-year terms, with the years for each class expiring in different years, if permitted by a company's certificate of incorporation, an initial bylaw or a bylaw adopted by the stockholders. A director elected to serve a term on such a classified board may not be removed by stockholders without cause. There is no limit in the number of terms a director may serve.

#### *Managing director and supervisory director vacancies*

*The Netherlands.* Under Dutch law, managing directors and supervisory directors are appointed by the general meeting of shareholders. Under our articles of association, managing directors and supervisory directors are appointed by the general meeting of shareholders upon the binding nomination by our supervisory board. However, the general meeting of shareholders, may at all times overrule such binding nomination by a resolution adopted by at least a two-thirds majority of the votes cast, provided such majority represents more than half of the issued share capital of our company. If the general meeting of shareholders overrules the binding nomination, the supervisory board must make a new nomination.

*Delaware.* The Delaware General Corporation Law provides that vacancies and newly created directorships may be filled by a majority of the directors then in office (even though less than a quorum) unless (1) otherwise provided in the certificate of incorporation or bylaws of the corporation or (2) the certificate of



incorporation directs that a particular class of stock is to elect such director, in which case any other directors elected by such class, or a sole remaining director elected by such class, will fill such vacancy.

### ***Conflict-of-interest transactions***

*The Netherlands.* Pursuant to Dutch law and our articles of association, managing directors and supervisory directors may not take part in any discussion or decision-making that involves a subject or transaction in relation to which it has a conflict of interest with us. Our articles of association provide that if as a result thereof no resolution of the management board can be adopted, the resolution will be adopted by the supervisory board. If as a result of a conflict of interest of supervisory directors no resolution of the supervisory board can be adopted, the resolution can nonetheless be adopted by the supervisory board as if there was no conflict of interest. In that case, each supervisory board member is entitled to participate in the discussion and decision making process and to cast a vote.

*Delaware.* The Delaware General Corporation Law generally permits transactions involving a Delaware corporation and an interested director of that corporation if:

- the material facts as to the director's relationship or interest are disclosed and a majority of disinterested directors consent;
- the material facts are disclosed as to the director's relationship or interest and a majority of shares entitled to vote thereon consent; or
- the transaction is fair to the corporation at the time it is authorized by the board of directors, a committee of the board of directors or the stockholders.

### ***Proxy voting by managing directors and supervisory directors***

*The Netherlands.* An absent managing director may issue a proxy for a specific management board meeting in writing but only to another management board member. An absent supervisory director may issue a proxy for a specific supervisory board meeting in writing but only to another supervisory board member.

*Delaware.* A director of a Delaware corporation may not issue a proxy representing the director's voting rights as a director.

### ***Shareholder rights***

#### *Voting rights*

*The Netherlands.* In accordance with Dutch law and our articles of association, each issued ordinary share confers the right to cast one vote at the general meeting of shareholders. Each holder of ordinary shares may cast as many votes as it holds shares. Shares that are held by us or our direct or indirect subsidiaries do not confer the right to vote. Dutch law does not permit cumulative voting for the election of managing directors and supervisory directors.

For each general meeting of shareholders, a record date will be applied with respect to ordinary shares in order to establish which shareholders are entitled to attend and vote at a specific general meeting of shareholders. Such record date is set by the management board. The record date and the manner in which shareholders can register and exercise their rights will be set out in the convocation notice of the meeting.

*Delaware.* Under the Delaware General Corporation Law, each stockholder is entitled to one vote per share of stock, unless the certificate of incorporation provides otherwise. In addition, the certificate of incorporation may provide for cumulative voting at all elections of directors of the corporation, or at elections held under specified circumstances. Either the certificate of incorporation or the bylaws may specify the number of shares and/or the amount of other securities that must be represented at a meeting in order to constitute a quorum, but in no event will a quorum consist of less than one third of the shares entitled to vote at a meeting.

Stockholders as of the record date for the meeting are entitled to vote at the meeting, and the board of directors may fix a record date that is no more than 60 nor less than ten days before the date of the meeting, and if no record date is set then the record date is the close of business on the day next preceding the day on which notice is given, or if notice is waived then the record date is the close of business on the day next preceding the day on which the meeting is held. The determination of the stockholders of record entitled to notice or to vote at a meeting of stockholders shall apply to any adjournment of the meeting, but the board of directors may fix a new record date for the adjourned meeting.

#### *Shareholder proposals*

*The Netherlands.* Pursuant to our articles of association, extraordinary general meetings of shareholders will be held whenever our supervisory board and/or our management board deem such to be necessary. Pursuant to Dutch law, one or more shareholders representing at least one-tenth of the issued share capital of the company may request the Dutch courts to order that a general meeting of shareholders be held and may, on their application, be authorized by the court to convene a general meeting of shareholders. The court shall disallow the application if it does not appear that the applicants have previously requested the management board and the supervisory board to convene a general meeting of shareholders and neither the management nor the supervisory board has taken the necessary steps so that the general meeting of shareholders could be held within six weeks after the request.

Also, the agenda for a general meeting of shareholders shall include such items requested by one or more shareholders and/or others entitled to attend general meetings of shareholders representing at least 3% of the issued share capital of a company or such lower percentage as the articles of association may provide. Our articles of association do not state such lower percentage.

*Delaware.* Delaware law does not specifically grant stockholders the right to bring business before an annual or special meeting. However, if a Delaware corporation is subject to the SEC's proxy rules, a stockholder who owns at least \$2,000 in market value, or 1% of the corporation's securities entitled to vote, may propose a matter for a vote at an annual or special meeting in accordance with those rules.

#### *Action by written consent*

*The Netherlands.* Under Dutch law, shareholders' resolutions may be adopted in writing without holding a meeting of shareholders, provided that (i) all shareholders agree on this practice for decision making and, (ii) the resolution is adopted unanimously by all shareholders that are entitled to vote. For a listed company, this method of adopting resolutions is not feasible.

*Delaware.* Although permitted by Delaware law, publicly listed companies do not typically permit stockholders of a corporation to take action by written consent.

#### *Appraisal rights*

*The Netherlands.* The concept of appraisal rights does not exist under Dutch law.

However, pursuant to Dutch law a shareholder who for his own account contributes at least 95% of our issued share capital may initiate proceedings against our minority shareholders jointly for the transfer of their shares to the claimant. The proceedings are held before the Enterprise Chamber (*ondernemingskamer*). The Enterprise Chamber may grant the claim for squeeze-out in relation to all minority shareholders and will determine the price to be paid for the shares, if necessary after appointment of one or three experts who will offer an opinion to the Enterprise Chamber on the value to be paid for the shares of the minority shareholders. For additional information, please see "Squeeze-out proceedings".

Furthermore, in accordance with directive 2005/56/EC of the European Parliament and the Council of October 26, 2005 on cross-border mergers of limited liability companies, Dutch law provides that, to the extent the acquiring company in a cross-border merger is organized under the laws of another EU member

state, a shareholder of a Dutch disappearing company who has voted against the cross-border merger may file a claim with the Dutch company for compensation. The compensation is to be determined by one or more independent experts.

**Delaware.** The Delaware General Corporation Law provides for stockholder appraisal rights, or the right to demand payment in cash of the judicially determined fair value of the stockholder's shares, in connection with certain mergers and consolidations.

#### *Shareholder suits*

**The Netherlands.** In the event a third party is liable to a Dutch company, only a company itself can bring a civil action against that third party. An individual shareholder does not have the right to bring an action on behalf of a company. This individual shareholder may, in its own name, have an individual right to take action against such third party in the event that the cause for the liability of that third party also constitutes a tortious act directly against that individual shareholder. The Dutch Civil Code provides for the possibility to initiate such action collectively. A collective action can be instituted by a foundation or an association whose objective is to protect the rights of a group of persons having similar interests. The collective action itself cannot result in an order for payment of monetary damages but may only result in a declaratory judgment (*verklaring voor recht*). In order to obtain compensation for damages, the foundation or association and the defendant may reach—often on the basis of such declaratory judgment—a settlement. A Dutch court may declare the settlement agreement binding upon all the injured parties with an opt-out choice for an individual injured party. An individual injured party may also itself—outside the collective action—institute a civil claim for damages.

**Delaware.** Under the Delaware General Corporation Law, a stockholder may bring a derivative action on behalf of the corporation to enforce the rights of the corporation. An individual also may commence a class action suit on behalf of himself and other similarly situated stockholders where the requirements for maintaining a class action under Delaware law have been met. A person may institute and maintain such a suit only if that person was a stockholder at the time of the transaction which is the subject of the suit. In addition, under Delaware case law, the plaintiff normally must be a stockholder at the time of the transaction that is the subject of the suit and throughout the duration of the derivative suit. Delaware law also requires that the derivative plaintiff make a demand on the directors of the corporation to assert the corporate claim before the suit may be prosecuted by the derivative plaintiff in court, unless such a demand would be futile.

#### **Repurchase of shares**

**The Netherlands.** Under Dutch law, a company such as ours may not subscribe for newly issued shares in its own capital. Such company may, however, subject to certain restrictions under Dutch law and its articles of association, acquire shares in its own capital. We may acquire fully paid shares in our own capital at any time for no valuable consideration. Furthermore, subject to certain provisions of Dutch law and our articles of association, we may repurchase fully paid-up shares in our own share capital if (1) such repurchase would not cause our shareholders' equity to fall below an amount equal to the sum of the paid-up and called-up part of the issued share capital and the reserves we are required to maintain pursuant to applicable law and (2) we would not as a result of such repurchase hold more than 50% of our own issued share capital.

Other than shares acquired for no valuable consideration, ordinary shares may only be acquired following a resolution of our management board, acting pursuant to an authorization for the repurchase of shares granted by the general meeting of shareholders. An authorization by the general meeting of shareholders for the repurchase of shares can be granted for a maximum period of 18 months. Such authorization must specify the number and class of shares that may be acquired, the manner in which these shares may be acquired and the price range within which the shares may be acquired. Our management board has been

authorized, acting with the approval of our supervisory board, for a period of 18 months to cause the repurchase of ordinary shares by us of up to 50% of our issued share capital, for a price per share not exceeding 110% of the average closing price of the ordinary shares on the NASDAQ Global Market for the five trading days prior to the day of purchase.

No authorization of the general meeting of shareholders is required if ordinary shares are acquired by us with the intention of transferring such ordinary shares to our employees under an applicable employee stock purchase plan.

*Delaware.* Under the Delaware General Corporation Law, a corporation may purchase or redeem its own shares unless the capital of the corporation is impaired or the purchase or redemption would cause an impairment of the capital of the corporation. A Delaware corporation may, however, purchase or redeem out of capital any of its preferred shares or, if no preferred shares are outstanding, any of its own shares if such shares will be retired upon acquisition and the capital of the corporation will be reduced in accordance with specified limitations.

### **Anti-takeover provisions**

*The Netherlands.* Under Dutch law, various protective measures are possible and permissible within the boundaries set by Dutch statutory law and Dutch case law. We have adopted several provisions that may have the effect of making a takeover of our company more difficult or less attractive, including:

- the authorization of a class of preference shares that may, subject to the approval of our supervisory board, be issued by our management board to a friendly party in such a manner as to dilute the interest of any potential acquirer;
- the staggered four-year terms of our supervisory directors, as a result of which only approximately one-fourth of our supervisory directors will be subject to election in any one year;
- a provision that our managing directors and supervisory directors may only be removed at the general meeting of shareholders by a two-thirds majority of votes cast representing more than half of our outstanding share capital if such removal is not proposed by our supervisory board; and
- requirements that certain matters, including an amendment of our articles of association, may only be brought to our shareholders for a vote upon a proposal by our management board that has been approved by our supervisory board.

*Delaware.* In addition to other aspects of Delaware law governing fiduciary duties of directors during a potential takeover, the Delaware General Corporation Law also contains a business combination statute that protects Delaware companies from hostile takeovers and from actions following the takeover by prohibiting some transactions once an acquirer has gained a significant holding in the corporation.

- Section 203 of the Delaware General Corporation Law prohibits "business combinations," including mergers, sales and leases of assets, issuances of securities and similar transactions by a corporation or a subsidiary with an interested stockholder that beneficially owns 15% or more of a corporation's voting stock, within three years after the person becomes an interested stockholder, unless: the transaction that will cause the person to become an interested stockholder is approved by the board of directors of the target prior to the transactions;
- after the completion of the transaction in which the person becomes an interested stockholder, the interested stockholder holds at least 85% of the voting stock of the corporation not including shares owned by persons who are directors and representatives of interested stockholders and shares owned by specified employee benefit plans; or
- after the person becomes an interested stockholder, the business combination is approved by the board of directors of the corporation and holders of at least 66.67% of the outstanding voting stock, excluding shares held by the interested stockholder.

A Delaware corporation may elect not to be governed by Section 203 by a provision contained in the original certificate of incorporation of the corporation or an amendment to the original certificate of incorporation or to the bylaws of the company, which amendment must be approved by a majority of the shares entitled to vote and may not be further amended by the board of directors of the corporation. Such an amendment is not effective until twelve months following its adoption.

### ***Inspection of books and records***

*The Netherlands.* Our management board and our supervisory board provide the shareholders, at the general meeting of shareholders, with all information that the shareholders require for the exercise of their powers, unless doing so would be contrary to an overriding interest of ours. Our management board or our supervisory board must give reason for electing not to provide such information on the basis of overriding interest.

*Delaware.* Under the Delaware General Corporation Law, any stockholder may inspect certain of the corporation's books and records, for any proper purpose, during the corporation's usual hours of business.

### ***Removal of managing directors and supervisory directors***

*The Netherlands.* Under our articles of association, the general meeting of shareholders, are at all times entitled to suspend or dismiss a managing director or supervisory director. The general meeting of shareholders may only adopt a resolution to suspend or dismiss such a member by at least a two-thirds majority of the votes cast, provided such majority represents more than half of the issued share capital of our company, unless the proposal was made by the supervisory board in which case a simple majority of the votes cast is sufficient.

*Delaware.* Under the Delaware General Corporation Law, any director or the entire board of directors may be removed, with or without cause, by the holders of a majority of the shares then entitled to vote at an election of directors, except (1) unless the certificate of incorporation provides otherwise, in the case of a corporation whose board is classified, stockholders may effect such removal only for cause, or (2) in the case of a corporation having cumulative voting, if less than the entire board is to be removed, no director may be removed without cause if the votes cast against his removal would be sufficient to elect him if then cumulatively voted at an election of the entire board of directors, or, if there are classes of directors, at an election of the class of directors of which he is a part.

### ***Preemptive rights***

*The Netherlands.* Under Dutch law, in the event of an issuance of ordinary shares, each shareholder will have a *pro rata* preemptive right in proportion to the aggregate nominal value of the ordinary shares held by such holder (with the exception of ordinary shares to be issued to employees or ordinary shares issued against a contribution other than in cash). Under our articles of association, the preemptive rights in respect of newly issued ordinary shares may be restricted or excluded by a resolution of the general meeting of shareholders upon proposal of our management board. The general meeting of shareholders may designate a corporate body, for example our management board, to restrict or exclude the preemptive rights in respect of newly issued ordinary shares, subject to the approval of our supervisory board. Such designation can be granted for a period not exceeding five years. A resolution of the general meeting of shareholders to restrict or exclude the preemptive rights or to designate the management board as the authorized body to do so requires a two-thirds majority of the votes cast, if less than one half of our issued share capital is represented at the meeting.

At our extraordinary general meeting held on \_\_\_\_\_ 2014, the general meeting of shareholders to authorize our management board acting with the approval of our supervisory board for a period of five years from \_\_\_\_\_, 2014 to limit or exclude preemptive rights accruing to shareholders in connection with the issue of ordinary shares or rights to subscribe for ordinary shares.

No preemptive rights apply in respect of preference shares.

*Delaware.* Under the Delaware General Corporation Law, stockholders have no preemptive rights to subscribe for additional issues of stock or to any security convertible into such stock unless, and to the extent that, such rights are expressly provided for in the certificate of incorporation.

## **Dividends**

*The Netherlands.* Dutch law provides that dividends may be distributed after adoption of the annual accounts by the general meeting of shareholders from which it appears that such dividend distribution is allowed. Moreover, dividends may be distributed only to the extent the shareholders' equity exceeds the amount of the paid-up and called-up part of the issued share capital of the company and the reserves that must be maintained under the law or the articles of association. Interim dividends may be declared as provided in the articles of association and may be distributed to the extent that the shareholders' equity exceeds the amount of the issued and paid-up and called-up part of the issued share capital in our company and the required legal reserves as described above as apparent from our financial statements. Under Dutch law, the articles of association may prescribe that the management board decides what portion of the profits is to be held as reserve.

Under our articles of association first a dividend is paid out of the profit, if available for distribution, on any preferred shares of which none will be outstanding or prior to completion of this offering. Any amount remaining out of the profit is carried to a reserve as our management board determines, subject to the approval of our supervisory board. After reservation by our management board of any profit, the remaining profit will be at the disposal of the shareholders. Our corporate policy is to only make a distribution of dividends to our shareholders after the adoption of our annual accounts demonstrating that such distribution is legally permitted. However, our management board is permitted, subject to certain requirements and subject to approval of the supervisory board, to declare interim dividends without the approval of the general meeting of shareholders.

Dividends and other distributions will be made payable not later than the date determined by the management board. Claims to dividends and other distribution not made within five years from the date that such dividends or distributions became payable will lapse and any such amounts will be considered to have been forfeited to us (*verjaring*).

*Delaware.* Under the Delaware General Corporation Law, a Delaware corporation may pay dividends out of its surplus (the excess of net assets over capital), or in case there is no surplus, out of its net profits for the fiscal year in which the dividend is declared and/or the preceding fiscal year (provided that the amount of the capital of the corporation is not less than the aggregate amount of the capital represented by the issued and outstanding stock of all classes having a preference upon the distribution of assets). In determining the amount of surplus of a Delaware corporation, the assets of the corporation, including stock of subsidiaries owned by the corporation, must be valued at their fair market value as determined by the board of directors, without regard to their historical book value. Dividends may be paid in the form of shares, property or cash.

## **Shareholder vote on certain reorganizations**

*The Netherlands.* Under Dutch law, the general meeting of shareholders must approve resolutions of the management board relating to a significant change in the identity or the character of the company or the business of the company, which includes:

- a transfer of the business or virtually the entire business to a third party;
- the entry into or termination of a long-term cooperation of the company or a subsidiary with another legal entity or company or as a fully liable partner in a limited partnership or general partnership, if such cooperation or termination is of a far-reaching significance for the company; and

- the acquisition or divestment by the company or a subsidiary of a participating interest in the capital of a company having a value of at least one third of the amount of its assets according to its balance sheet and explanatory notes or, if the company prepares a consolidated balance sheet, according to its consolidated balance sheet and explanatory notes, in the last adopted annual accounts of the company.

Under Dutch law, a shareholder who owns shares representing at least 95% of the nominal value of a company's issued share capital may institute proceedings against the company's other shareholders jointly for the transfer of their shares to that shareholder. For additional information, please see "Squeeze-out proceedings".

*Delaware.* Under the Delaware General Corporation Law, the vote of a majority of the outstanding shares of capital stock entitled to vote thereon generally is necessary to approve a merger or consolidation or the sale of all or substantially all of the assets of a corporation. The Delaware General Corporation Law permits a corporation to include in its certificate of incorporation a provision requiring for any corporate action the vote of a larger portion of the stock or of any class or series of stock than would otherwise be required.

Under the Delaware General Corporation Law, no vote of the stockholders of a surviving corporation to a merger is needed, however, unless required by the certificate of incorporation, if (1) the agreement of merger does not amend in any respect the certificate of incorporation of the surviving corporation, (2) the shares of stock of the surviving corporation are not changed in the merger and (3) the number of shares of common stock of the surviving corporation into which any other shares, securities or obligations to be issued in the merger may be converted does not exceed 20% of the surviving corporation's common stock outstanding immediately prior to the effective date of the merger. In addition, stockholders may not be entitled to vote in certain mergers with other corporations that own 90% or more of the outstanding shares of each class of stock of such corporation, but the stockholders will be entitled to appraisal rights.

#### ***Remuneration of managing directors and supervisory directors***

*The Netherlands.* Under Dutch law and our articles of association, we must adopt a remuneration policy for managing directors. Such remuneration policy shall be adopted by the general meeting of shareholders upon the proposal of our supervisory board. The supervisory board determines the remuneration of the managing directors in accordance with the remuneration policy. A proposal by the supervisory board with respect to remuneration schemes in the form of shares or rights to shares is submitted for approval by the supervisory board to the general meeting of shareholders. Such proposal must set out at least the maximum number of shares or rights to shares to be granted to the management board and the criteria for granting such shares.

The general meeting of shareholders, may determine the remuneration of supervisory directors. The supervisory directors will be reimbursed for their expenses.

*Delaware.* Under the Delaware General Corporation Law, the stockholders do not generally have the right to approve the compensation policy for directors or the senior management of the corporation, although certain aspects of executive compensation may be subject to binding or advisory stockholder votes due to the provisions of U.S. federal securities and tax law, as well as stock exchange requirements.

#### **Trading Facility**

From 2007 until 2012, the ordinary shares of our predecessor entity, AMT, were listed on Euronext Amsterdam. Following our corporate reorganization announced in February 2012 and completed in April 2012, pursuant to which uniQure acquired the entire business and assets of AMT, the ordinary shares of AMT were delisted from that exchange. In connection with our corporate reorganization, our shareholders other than our significant shareholders received depositary receipts representing our class B ordinary shares, which are held on their behalf by the Stichting Administratiekantoor uniQure BV, or STAK, an independent

Dutch foundation we formed for that purpose. To provide an opportunity for liquidity for these former public shareholders of AMT, the STAK established a limited trading facility for our depositary receipts on the Nederlandsche Participatie Exchange, or NPEX, an electronic, auction-based trading platform in the Netherlands. We bear the costs of this facility. NPEX operates periodic auctions, currently once per month, in the securities of included companies, but is not an established market. We suspended further trading in our depositary receipts through this facility in November 2013 in anticipation of this offering.



## SHARES ELIGIBLE FOR FUTURE SALE

Upon completion of this offering, we will have outstanding \_\_\_\_\_ ordinary shares. All of the ordinary shares sold in this offering will be freely transferable by persons other than our "affiliates" without restriction or further registration under the Securities Act. Sales of substantial numbers of our ordinary shares in the public market could adversely affect prevailing market prices of our ordinary shares. Prior to this offering, there has been no public market for our ordinary shares, and while application has been made for the ordinary shares to be quoted on the NASDAQ Global Market, we cannot assure you that a regular trading market will develop in the ordinary shares.

## Rule 144

In general, under Rule 144 of the Securities Act, beginning 90 days after the date of this prospectus, an "affiliate" who has beneficially owned our shares for a period of at least six months is entitled to sell within any three-month period a number of shares that does not exceed the greater of either 1% of our then outstanding shares, or approximately \_\_\_\_\_ shares immediately after this offering, or the average weekly trading volume of our shares on the NASDAQ Global Market during the four calendar weeks preceding the filing with the SEC of a notice on Form 144 with respect to such sale. Such sales under Rule 144 of the Securities Act are also subject to prescribed requirements relating to the manner of sale, notice and availability of current public information about us.

Under Rule 144, a person who is not deemed to have been an affiliate of ours at any time during the 90 days preceding a sale, and who has beneficially owned the shares proposed to be sold for at least six months, including the holding period of any prior holder other than an affiliate, is entitled to sell such shares without restriction, provided we have been in compliance with our reporting requirements under the Exchange Act for the six months following satisfaction of the six-month holding period. To the extent that our affiliates sell their shares, other than pursuant to Rule 144 or a registration statement, the purchaser's holding period for the purpose of effecting a sale under Rule 144 commences on the date of transfer from the affiliate.

## Rule 701

In general, under Rule 701 of the Securities Act, each of our employees, consultants or advisors who purchases our ordinary shares from us in connection with a compensatory stock plan or other written agreement executed prior to the completion of this offering is eligible to resell such ordinary shares in reliance on Rule 144, but without compliance with some of the restrictions, including the holding period, contained in Rule 144.

## Regulation S

Regulation S provides generally that sales made in offshore transactions are not subject to the registration or prospectus-delivery requirements of the Securities Act.

## Lock-up Arrangements

For a description of the lock-up arrangements that we, the members of our management and supervisory boards and our shareholders have entered into in connection with this offering, see "Underwriting."

## TAXATION

### Taxation in the Netherlands

*The information given below is neither intended as tax advice nor purports to describe all of the tax considerations that may be relevant to a prospective holder of our ordinary shares. All statements as to matters of law and legal conclusions, but not as to factual matters, contained in this discussion, unless otherwise noted, are the opinions of Van Campen Liem (Liem & Partners N.V.) and are based on the accuracy of representations made by us. Prospective holders of ordinary shares are advised to consult their tax counsel with respect to the tax consequences of acquiring, holding and/or disposing of ordinary shares.*

This taxation summary solely addresses the principal Dutch tax consequences of the acquisition, ownership and disposal of ordinary shares. It does not purport to describe all the tax considerations that may be relevant to a prospective holder of our ordinary shares, or a Shareholder. Where in this summary English terms and expressions are used to refer to Dutch concepts, the meaning to be attributed to such terms and expressions shall be the meaning to be attributed to the equivalent Dutch concepts under Dutch tax law.

*This summary does not address the tax consequences of:*

- A Shareholder who is an individual, either resident or non-resident in the Netherlands, and who has a substantial interest (*aanmerkelijk belang*) in us within the meaning of the Dutch Income Tax Act 2001 (*Wet inkomstenbelasting 2001*). Generally, if a person holds an interest in us, such interest forms part of a substantial interest, or a deemed substantial interest, in us, if any or more of the following circumstances is present:
  - If a Shareholder, either alone or, in the case of an individual, together with his partner owns or is deemed to own, directly or indirectly, either a number of shares in us representing five percent or more of our total issued and outstanding capital (or the issued and outstanding capital of any class of our shares), or rights to acquire, directly or indirectly, shares, whether or not already issued, representing five percent or more of our total issued and outstanding capital (or the issued and outstanding capital of any class of our shares), or profit participating certificates (*winstbewijzen*), relating to five percent or more of our annual profit or to five percent of our liquidation proceeds.
  - If the shares, profit participating certificates or rights to acquire shares in us are held or deemed to be held following the application of a non-recognition provision.
  - If the partner of a Shareholder, or one of certain relatives of the Shareholder or of this partner has a substantial interest (as described under 1. and 2. above) in us.
  - A Shareholder receiving income or realizing capital gains in their capacity as future, present or past employee (*werknemer*) or member of a management board (*bestuurder*), or supervisory director (*commissaris*).
  - Pension funds, investment institutions (*fiscale beleggingsinstellingen*), exempt investment institutions (*vrijgestelde beleggingsinstellingen*) and other entities that are exempt from corporate income tax in the Netherlands, as well as entities that are exempt from corporate income tax in their country of residence, such country of residence being another state of the European Union, Norway, Liechtenstein, Iceland or any other state with which the Netherlands have agreed to exchange information in line with international standards.

For purposes of Dutch personal income tax and Dutch corporate income tax, ordinary shares legally owned by a third party, such as a trustee, foundation or similar entity or arrangement, may under certain circumstances have to be allocated to the (deemed) settler, grantor or similar organiser or, upon the death of the Settlor, his/her beneficiaries in proportion to their entitlement to the estate of the Settlor of such trust or similar arrangement.

This summary is based on the tax laws and principles (unpublished case law not included) in the Netherlands as in effect on the date of this prospectus, which are subject to changes that could prospectively or retroactively affect the stated tax consequences. Where in this summary the terms "the Netherlands" and "Dutch" are used, these refer solely to the European part of the Kingdom of the Netherlands.

## **Dividend Withholding Tax**

### *General*

We are generally required to withhold Dutch dividend withholding tax at a rate of 15% from dividends distributed by us. The concept dividends "distributed by us" as used in this section includes, but is not limited to:

- distributions of profits in cash or in kind, whatever they may be named or in whatever form;
- liquidation proceeds, or proceeds from the repurchase of ordinary shares by us in excess of the average paid-in capital recognized for Dutch dividend withholding tax purposes;
- the par value of ordinary shares issued to a Shareholder in us or an increase of the par value of ordinary shares, to the extent that it does not appear that a contribution, recognized for Dutch dividend withholding tax purposes, has been made or will be made; and
- partial repayment of share capital, if and to the extent that there are net profits (zuivere winst), unless (a) the general meeting of shareholders has resolved in advance to make such repayment and (b) the par value of the shares concerned has been reduced by an equal amount by way of an amendment to our articles of association.

In general, we will be required to remit all amounts withheld as Dutch dividend withholding tax to the Dutch tax authorities. However, under certain circumstances, we are allowed to reduce the amount to be remitted to the Dutch tax authorities by the lesser of:

- 3% of the portion of the distribution paid by us that is subject to Dutch dividend withholding tax; and
- 3% of the dividends and profit distributions, before deduction of foreign withholding taxes, received by us from qualifying foreign subsidiaries in the current calendar year (up to the date of the distribution by us) and the two preceding calendar years, as far as such dividends and profit distributions have not yet been taken into account for purposes of establishing the above mentioned reduction.

Although this reduction reduces the amount of Dutch dividend withholding tax that we are required to remit to the Dutch tax authorities, it does not reduce the amount of tax that we are required to withhold on dividends distributed.

### *Residents of the Netherlands*

A Shareholder which is resident or deemed resident in the Netherlands is generally entitled to a full credit of any Dutch dividend withholding tax against the Dutch (corporate) income tax liability of such Shareholder, and is generally entitled to a refund in the form of a negative assessment of Dutch (corporate) income tax, insofar such Dutch dividend withholding tax, together with any other creditable domestic and/or foreign taxes, exceeds such Shareholder's aggregate Dutch income tax or Dutch corporate income tax liability.

If and to the extent that such a corporate Shareholder is eligible for the application of the participation exemption with respect to the ordinary shares, dividends distributed by us are in principle exempt from Dutch dividend withholding tax.

Pursuant to domestic anti-dividend stripping rules, no exemption from Dutch dividend withholding tax, credit against Dutch (corporate) income tax, refund or reduction of Dutch dividend withholding tax shall apply if the recipient of the dividend paid by us is not considered to be the beneficial owner (*uiteindelijk gerechtigde*) as meant in these rules, of such dividends.

*Non-residents of the Netherlands (including but not limited to U.S. Shareholders)*

A non-resident Shareholder, which is resident in the non-European part of the Kingdom of the Netherlands or in a country that has concluded a tax treaty with the Netherlands, may be eligible for a full or partial relief from Dutch dividend withholding tax, provided such relief is timely and duly claimed.

In addition, a non-resident Shareholder that is not an individual, is entitled to an exemption from Dutch dividend withholding tax, provided that each of the following tests are satisfied:

- the non-resident Shareholder is, according to the tax law of a Member State of the European Union or a state designated by a ministerial decree, that is a party to the Agreement regarding the European Economic Area, resident there and it is not transparent for tax purposes according to the tax law of such state;
- anyone or more of the following threshold conditions are satisfied:
  - at the time the dividend is distributed by us, the non-resident Shareholder holds shares representing at least five percent of our nominal paid-up capital; or
  - the non-resident Shareholder has held shares representing at least five percent of our nominal paid-up capital for a continuous period of more than one year at any time during four years preceding the time the dividend is distributed by us; or
  - the non-resident Shareholder is connected with us within the meaning of article 10a, paragraph 4 of the Dutch Corporate Income Tax Act 1969 (Wet op de vennootschapsbelasting 1969, or CITA); or
  - an entity connected with the non-resident Shareholder within the meaning of article 10a, paragraph 4 of CITA holds at the time of the dividends distributed by us, shares representing at least five per cent of our nominal paid-up capital; and
- the non-resident Shareholder is not considered to be resident outside the Member States of the European Union or the states designated by ministerial decree, that are party to the Agreement regarding the European Economic Area, under the terms of a tax treaty concluded with a third state.

A non-resident Shareholder which is resident in a Member State of the European Union with which the Netherlands has concluded a tax treaty that provides for a reduction of Dutch tax on dividends based on the ownership of the number of voting rights, the test under 2.a. above is also satisfied if the non-resident Shareholder owns at least five percent of the voting rights in us.

The exemption from Dutch dividend withholding tax is not available to a non-resident Shareholder if pursuant to a provision for the prevention of fraud or abuse included in a tax treaty between the Netherlands and the country of residence of the non-resident Shareholder, the non-resident Shareholder is not entitled to the reduction of Dutch tax on dividends provided for by such treaty.

Furthermore, pursuant to domestic anti-dividend stripping rules, no exemption from Dutch dividend withholding tax, refund or reduction of Dutch dividend withholding tax shall apply if the recipient of the dividend paid by us is not considered to be the beneficial owner (*uiteindelijk gerechtigde*) as meant in these rules, of such dividends. The Dutch tax authorities have taken the position that this beneficial ownership test can also be applied to deny relief from Dutch dividend withholding tax under tax treaties and the Tax Arrangement for the Kingdom (*Belastingregeling voor het Koninkrijk*).

A non-resident Shareholder which is subject to Dutch income tax or Dutch corporate income tax in respect of any benefits derived or deemed to be derived from ordinary shares, including any capital gain realized on

the disposal thereof, can generally credit Dutch dividend withholding tax against its Dutch income tax or its Dutch corporate income tax liability, as applicable, and is generally entitled to a refund pursuant to a negative tax assessment if and to the extent the Dutch dividend withholding tax, together with any other creditable domestic and/or foreign taxes, exceeds its aggregate Dutch income tax or its aggregate Dutch corporate income tax liability, respectively.

## **Taxes on Income and Capital Gains**

### *Residents of the Netherlands*

#### *Individuals*

A Shareholder, who is an individual resident or deemed to be resident in the Netherlands, or who has elected to be taxed as a resident of the Netherlands for Dutch personal income tax purposes, will be subject to regular Dutch personal income tax at progressive rates (up to a maximum rate of 52%) under the Dutch Income Tax Act 2001 on the income derived from the ordinary shares and gains realized on the disposal thereof if:

- such Shareholder derives any benefits from the ordinary shares, which are attributable to an enterprise of such Shareholder, whether as an entrepreneur or pursuant to a co-entitlement to the net worth of an enterprise, other than as a shareholder or an entrepreneur; or
- such income or gain is taxable in the hands of such Shareholder as benefits from miscellaneous activities (*resultaat uit overige werkzaamheden*), including but not limited to activities with respect to the ordinary shares that are beyond the scope of regular active portfolio management activities.

If neither of the two abovementioned conditions apply, such Shareholder must determine his or her taxable income with regard to the ordinary shares on the basis of a deemed return on income from savings and investments (*sparen en beleggen*), rather than on the basis of income actually received or gains actually realized. This deemed return on income from savings and investments has been fixed at a rate of 4% of the individual's yield basis at the beginning of the calendar year, insofar as the individual's yield basis exceeds a certain threshold. The individual's yield basis is determined as the fair market value of certain qualifying assets held by the individual less the fair market value of certain qualifying liabilities at the beginning of the calendar year.

#### *Corporate entities*

Generally, corporate Shareholders that are resident or deemed to be resident in the Netherlands for Dutch corporate income tax purposes will be subject to regular Dutch corporate income tax, levied at a rate of 25% (20% over profits up to €200,000) over income derived from the ordinary shares and gains realized upon acquisition, redemption and disposal of ordinary shares.

If and to the extent that such Shareholder is eligible for the application of the participation exemption with respect to the ordinary shares, income derived from the ordinary shares and gains and losses (with the exception of liquidation losses under strict conditions) realized on the ordinary shares may be exempt from Dutch corporate income tax.

### *Non-residents of the Netherlands (including but not limited to U.S. Shareholders)*

#### *Individuals*

A Shareholder, who is an individual not resident or deemed to be resident in the Netherlands, and who has not elected to be taxed as a resident of the Netherlands for Dutch income tax purposes, will not be subject to any Dutch taxes on income or capital gains in respect of dividends distributed by us or in respect of any gain realized on the disposal of ordinary shares (other than dividend withholding tax as described above), except if:

- such holder has an enterprise or an interest in an enterprise that is, in whole or in part, carried on through a permanent establishment or a permanent representative in the Netherlands and to which enterprise or part of an enterprise, as the case may be, the ordinary shares are attributable; or

- such income or gain such income or gain is taxable in the hands of such Shareholder as benefits from miscellaneous activities" (resultaat uit overige werkzaamheden), including but not limited to activities with respect to the ordinary shares that are beyond the scope of regular active portfolio management.

If one of the two abovementioned conditions apply, the income or gains in respect of dividends distributed by us or in respect of any capital gain realized on the disposal of ordinary shares will in general be subject to Dutch personal income tax at the progressive rates up to 52%.

#### *Corporate entities*

A corporate Shareholder, which is not resident or deemed to be resident in the Netherlands for Dutch corporate income tax purposes, will not be subject to any Dutch taxes on income or capital gains in respect of dividends distributed by us, or in respect of any gain realized, on the disposal of ordinary shares (other than dividend withholding tax as described above), except if:

- such Shareholder has an enterprise or an interest in an enterprise that is, in whole or in part, carried on through a permanent establishment or a permanent representative in the Netherlands, to which the ordinary shares are attributable; or
- such holder has a substantial interest or a deemed substantial interest in us (as described above) , that (i) is held with the evasion of income tax or dividend withholding tax as (one of) the main purpose(s) and (ii) is not attributable to the assets of an enterprise of such Shareholder; or
- such holder is an entity resident of Aruba, Curaçao or Saint Martin with a permanent establishment or permanent representative in Bonaire, Saint Eustatius or Saba to which such income or gain is attributable, and the permanent establishment or permanent representative would be deemed to be resident of the Netherlands for Dutch corporate income tax purposes (i) had the permanent establishment been a corporate entity (lichaam), or (ii) had the activities of the permanent representative been conducted by a corporate entity, respectively.

If one of the abovementioned conditions applies, income derived from the ordinary shares and gains realized on ordinary shares will, in general, be subject to regular Dutch corporate income tax levied at a rate of 25% (20% over profits up to €200,000), except that a holder referred to under (2) above will generally be subject to an effective corporate income tax rate of 15% if it holds the substantial interest in us only with the purpose of avoiding dividend withholding tax and not with (one of) the main purposes to avoid income tax.

#### **Gift or Inheritance Taxes**

No Dutch gift or Dutch inheritance tax is due in respect of any gift, in form or in substance, of the ordinary shares by, or inheritance of the shares on the death of, a Shareholder except if:

- at the time of the gift or death of the Shareholder, the Shareholder is resident, or deemed to be resident, in the Netherlands for purposes of Dutch gift tax or Dutch inheritance tax, as applicable; or
- in the case of a gift of ordinary shares by an individual who at the date of the gift was neither resident nor deemed to be resident in the Netherlands (i) such individual dies within 180 days after the date of the gift, while being resident or deemed to be resident in the Netherlands; or (ii) the gift of ordinary shares is made under a condition precedent and the Shareholder is resident, or is deemed to be resident in the Netherlands at the time the condition is fulfilled.

For purposes of the above, a gift of ordinary shares made under a condition precedent (*opschortende voorwaarde*) is deemed to be made at the time the condition precedent is satisfied.

For purposes of Dutch gift or Dutch inheritance taxes, an individual not holding the Dutch nationality will be deemed to be resident in the Netherlands, *inter alia*, if he or she has been resident in the Netherlands at any time during the ten years preceding the date of the gift or his or her death. Additionally, for purposes

of Dutch gift tax, an individual not holding the Dutch nationality will be deemed to be resident in the Netherlands if he or she has been resident in the Netherlands at any time during the twelve months preceding the date of the gift. Applicable tax treaties may override deemed residency in the Netherlands.

### Value Added Tax

No Dutch value added tax will arise in respect of payments in consideration for the issue, acquisition, ownership and disposal of ordinary shares, other than value added taxes on fees payable in respect of services not exempt from Dutch value added tax.

### Other Taxes and Duties

No Dutch registration tax, capital tax, custom duty, transfer tax, stamp duty or any other similar tax or duty, other than court fees, will be payable in the Netherlands in respect of or in connection with the subscription, issue, placement, allotment, delivery or transfer of the ordinary shares.

### Residence

A Shareholder will not become resident, or deemed resident in the Netherlands for tax purposes by reason only of holding the ordinary shares.

### Taxation in the United States

The following summary of the material U.S. federal income tax consequences of the acquisition, ownership and disposition of our ordinary shares is based upon current law and does not purport to be a comprehensive discussion of all the tax considerations that may be relevant to a decision to purchase our ordinary shares. This summary is based on current provisions of the Code, existing, final, temporary and proposed United States Treasury Regulations, administrative rulings and judicial decisions, in each case as available on the date of this prospectus. All of the foregoing are subject to change, which change could apply retroactively and could affect the tax consequences described below.

This section summarizes the material U.S. federal income tax consequences to U.S. holders, as defined below, of ordinary shares. This summary addresses only the U.S. federal income tax considerations for U.S. holders that acquire the ordinary shares at their original issuance and hold the ordinary shares as capital assets. This summary does not address all U.S. federal income tax matters that may be relevant to a particular U.S. holder. **Each prospective investor should consult a professional tax advisor with respect to the tax consequences of the acquisition, ownership or disposition of the ordinary shares.** This summary does not address tax considerations applicable to a holder of ordinary shares that may be subject to special tax rules including, without limitation, the following:

- certain financial institutions;
- insurance companies;
- dealers or traders in securities, currencies, or notional principal contracts;
- tax-exempt entities;
- regulated investment companies;
- persons that hold the ordinary shares as part of a hedge, straddle, conversion, constructive sale or similar transaction involving more than one position;
- persons that hold the ordinary shares through partnerships or certain other pass-through entities;
- holders (whether individuals, corporations or partnerships) that are treated as expatriates for some or all U.S. federal income tax purposes;
- holders that own (or are deemed to own) 10% or more of our voting shares; and
- holders that have a "functional currency" other than the U.S. dollar.

Further, this summary does not address alternative minimum tax consequences or the indirect effects on the holders of equity interests in entities that own our ordinary shares. In addition, this discussion does not consider the U.S. tax consequences to holders of ordinary shares that are not "U.S. holders" (as defined below).

For the purposes of this summary, a "U.S. holder" is a beneficial owner of ordinary shares that is (or is treated as), for U.S. federal income tax purposes:

- an individual who is either a citizen or resident of the United States;
- a corporation, or other entity that is treated as a corporation for U.S. federal income tax purposes, created or organized in or under the laws of the United States or any state of the United States or the District of Columbia;
- an estate, the income of which is subject to U.S. federal income taxation regardless of its source; or
- a trust, if a court within the United States is able to exercise primary supervision over its administration and one or more U.S. persons have the authority to control all of the substantial decisions of such trust or has a valid election in effect under applicable U.S. Treasury Regulations to be treated as a United States person.

If a partnership holds ordinary shares, the tax treatment of a partner will generally depend upon the status of the partner and upon the activities of the partnership.

We will not seek a ruling from the U.S. Internal Revenue Service ("IRS") with regard to the U.S. federal income tax treatment of an investment in our ordinary shares, and we cannot assure you that that the IRS will agree with the conclusions set forth below.

*Distributions.* Subject to the discussion under "*Passive Foreign Investment Company Considerations*" below, the gross amount of any distribution (including any amounts withheld in respect of Dutch withholding tax) actually or constructively received by a U.S. holder with respect to ordinary shares will be taxable to the U.S. holder as a dividend to the extent of our current and accumulated earnings and profits as determined under U.S. federal income tax principles. Distributions in excess of earnings and profits will be non-taxable to the U.S. holder to the extent of, and will be applied against and reduce, the U.S. holder's adjusted tax basis in the ordinary shares. Distributions in excess of earnings and profits and such adjusted tax basis will generally be taxable to the U.S. holder as capital gain from the sale or exchange of property. However, since we do not calculate our earnings and profits under U.S. federal income tax principles, it is expected that any distribution will be reported as a dividend, even if that distribution would otherwise be treated as a non-taxable return of capital or as capital gain under the rules described above. The amount of any distribution of property other than cash will be the fair market value of that property on the date of distribution. The U.S. holder will not be eligible for any dividends-received deduction in respect of the dividend otherwise allowable to corporations.

Under the Code and subject to the discussion below regarding the "Medicare tax," qualified dividends received by non-corporate U.S. holders (*i.e.*, individuals and certain trusts and estates) are subject to a maximum income tax rate of 20%. This reduced income tax rate is applicable to dividends paid by "qualified foreign corporations" to such non-corporate U.S. holders that meet the applicable requirements, including a minimum holding period (generally, at least 61 days during the 121-day period beginning 60 days before the ex-dividend date). We expect to be considered a qualified foreign corporation under the Code. Accordingly, dividends paid by us to non-corporate U.S. holders with respect to shares that meet the minimum holding period and other requirements are expected to be treated as "qualified dividend income." However, dividends paid by us will not qualify for the 20% maximum U.S. federal income tax rate if we are treated, for the tax year in which the dividends are paid or the preceding tax year, as a "passive foreign investment company" for U.S. federal income tax purposes, as discussed below.



Dividends received by a U.S. holder with respect to ordinary shares generally will be treated as foreign source income for the purposes of calculating that holder's foreign tax credit limitation. Subject to applicable conditions and limitations, and subject to the discussion in the next paragraph, any Dutch income tax withheld on dividends may be deducted from taxable income or credited against a U.S. holder's U.S. federal income tax liability. The limitation on foreign taxes eligible for the U.S. foreign tax credit is calculated separately with respect to specific classes of income. For this purpose, dividends distributed by us generally will constitute "passive category income" (but, in the case of some U.S. holders, may constitute "general category income").

Upon making a distribution to shareholders, we may be permitted to retain a portion of the amounts withheld as Dutch dividend withholding tax. See "—Taxation in the Netherlands—Dividend Withholding Tax—General." The amount of Dutch withholding tax that we may retain reduces the amount of dividend withholding tax that we are required to pay to the Dutch tax authorities but does not reduce the amount of tax we are required to withhold from dividends paid to U.S. holders. In these circumstances, it is likely that the portion of dividend withholding tax that we are not required to pay to the Dutch tax authorities with respect to dividends distributed to U.S. holders would not qualify as a creditable tax for U.S. foreign tax credit purposes.

*Sale or other disposition of ordinary shares.* A U.S. holder will generally recognize gain or loss for U.S. federal income tax purposes upon the sale or exchange of ordinary shares in an amount equal to the difference between the U.S. dollar value of the amount realized from such sale or exchange and the U.S. holder's tax basis for those ordinary shares. Subject to the discussion under "*Passive Foreign Investment Company Considerations*" below, this gain or loss will generally be a capital gain or loss and will generally be treated as from sources within the United States. Such capital gain or loss will be treated as long-term capital gain or loss if the U.S. holder has held the ordinary shares for more than one year at the time of the sale or exchange. Long-term capital gains of non-corporate holders may be eligible for a preferential tax rate; the deductibility of capital losses is subject to limitations.

*Medicare Tax.* An additional 3.8% tax is imposed on the net investment income (which includes taxable dividends and net capital gains) received by U.S. holders that are individuals, certain trusts or estates.

*Passive foreign investment company considerations.* A corporation organized outside the United States generally will be classified as a passive foreign investment company ("PFIC") for U.S. federal income tax purposes in any taxable year in which, after applying the applicable look-through rules, either: (i) at least 75% of its gross income is passive income, or (ii) on average at least 50% of the gross value of its assets is attributable to assets that produce passive income or are held for the production of passive income. In arriving at this calculation, a pro rata portion of the income and assets of each corporation in which we own, directly or indirectly, at least a 25% interest, as determined by the value of such corporation, must be taken into account. Passive income for this purpose generally includes dividends, interest, royalties, rents and gains from commodities and securities transactions.

We believe that we were not a PFIC for the 2012 taxable year. Based on our estimated gross income, the average value of our gross assets, and the nature of the active businesses conducted by our "25% or greater" owned subsidiaries, we do not believe that we will be classified as a PFIC in the current taxable year and do not expect to become one in the foreseeable future. Our status for any taxable year will depend on our assets and activities in each year, and because this is a factual determination made annually after the end of each taxable year, there can be no assurance that we will not be considered a PFIC for the current taxable year or any future taxable year. The market value of our assets may be determined in large part by reference to the market price of our ordinary shares, which is likely to fluctuate after the offering (and may fluctuate considerably given that market prices of technology companies have been especially volatile). In addition, the composition of our income and assets will be affected by how, and how quickly, we spend the cash we raise in this offering.

If we were a PFIC for any taxable year during which a U.S. holder held ordinary shares, under the "default PFIC regime" (i.e., in the absence of one of the elections described below) gain recognized by the U.S. holder on a sale or other disposition (including a pledge) of the ordinary shares would be allocated ratably over the U.S. holder's holding period for the ordinary shares. The amounts allocated to the taxable year of the sale or other disposition and to any year before we became a PFIC would be taxed as ordinary income. The amount allocated to each other taxable year would be subject to tax at the highest rate in effect for individuals or corporations, as appropriate, for that taxable year, and an interest charge would be imposed on the resulting tax liability for that taxable year. Similar rules would apply to the extent any distribution in respect of ordinary shares exceeds 125% of the average of the annual distributions on ordinary shares received by a U.S. holder during the preceding three years or the holder's holding period, whichever is shorter.

In the event we were treated as a PFIC, the tax consequences under the default PFIC regime described above could be avoided by either a "mark-to-market" or "qualified electing fund" election. A U.S. holder making a mark-to-market election generally would not be subject to the PFIC rules discussed above, except with respect to any portion of the holder's holding period that precedes the effective date of the election. Instead, the electing holder would include in ordinary income, for each taxable year in which we were a PFIC, an amount equal to any excess of (a) the fair market value of the ordinary shares as of the close of such taxable year over (b) the electing holder's adjusted tax basis in such ordinary shares. In addition, an electing holder would be allowed a deduction in an amount equal to the lesser of (a) the excess, if any, of (i) the electing holder's adjusted tax basis in the ordinary shares over (ii) the fair market value of such ordinary shares as of the close of such taxable year or (b) the excess, if any, of (i) the amount included in ordinary income because of the election for prior taxable years over (ii) the amount allowed as a deduction because of the election for prior taxable years. The election would cause adjustments in the electing holder's tax basis in the ordinary shares to reflect the amount included in gross income or allowed as a deduction because of the election. In addition, upon a sale or other taxable disposition of ordinary shares, an electing holder would recognize ordinary income or loss (not to exceed the excess, if any, of (a) the amount included in ordinary income because of the election for prior taxable years over (b) the amount allowed as a deduction because of the election for prior taxable years).

Alternatively, a U.S. holder making a valid and timely "QEF election" generally would not be subject to the default PFIC regime discussed above. Instead, for each PFIC year to which such an election applied, the electing holder would be subject to U.S. federal income tax on the electing holder's pro rata share of our net capital gain and ordinary earnings, regardless of whether such amounts were actually distributed to the electing holder. However, because we do not intend to prepare or provide the information that would permit the making of a valid QEF election, that election will not be available to U.S. holders.

If we were considered a PFIC for the current taxable year or any future taxable year, a U.S. holder would be required to file annual information returns for such year, whether or not the U.S. holder disposed of any ordinary shares or received any distributions in respect of ordinary shares during such year.

**Backup Withholding and Information Reporting.** U.S. holders generally will be subject to information reporting requirements with respect to dividends on ordinary shares and on the proceeds from the sale, exchange or disposition of ordinary shares that are paid within the United States or through U.S.-related financial intermediaries, unless the U.S. holder is an "exempt recipient." In addition, U.S. holders may be subject to backup withholding (at a 28% rate) on such payments, unless the U.S. holder provides a taxpayer identification number and a duly executed IRS Form W-9 or otherwise establishes an exemption. Backup withholding is not an additional tax, and the amount of any backup withholding will be allowed as a credit against a U.S. holder's U.S. federal income tax liability and may entitle such holder to a refund, provided that the required information is timely furnished to the IRS.

## UNDERWRITING

Subject to the terms and conditions set forth in the underwriting agreement, dated \_\_\_\_\_, 2014, between us and Jefferies LLC and Leerink Swann LLC, as the representatives of the underwriters named below and the joint book-running managers of this offering, we have agreed to sell to the underwriters, and each of the underwriters has agreed, severally and not jointly, to purchase from us, the respective number of ordinary shares shown opposite its name below:

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UNDERWRITER	NUMBER OF ORDINARY SHARES
Jefferies LLC	
Leerink Swann LLC	
Piper Jaffray & Co.	
Total	

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The underwriting agreement provides that the obligations of the several underwriters are subject to certain conditions precedent such as the receipt by the underwriters of officers' certificates and legal opinions and approval of certain legal matters by their counsel. The underwriting agreement provides that the underwriters will purchase all of the ordinary shares if any of them are purchased. If an underwriter defaults, the underwriting agreement provides that the purchase commitments of the non-defaulting underwriters may be increased or the underwriting agreement may be terminated. We have agreed to indemnify the underwriters and certain of their controlling persons against certain liabilities, including liabilities under the Securities Act, and to contribute to payments that the underwriters may be required to make in respect of those liabilities.

The underwriters have advised us that, following the completion of this offering, they currently intend to make a market in the ordinary shares as permitted by applicable laws and regulations. However, the underwriters are not obligated to do so, and the underwriters may discontinue any market-making activities at any time without notice in their sole discretion. Accordingly, no assurance can be given as to the liquidity of the trading market for the ordinary shares, that you will be able to sell any of the ordinary shares held by you at a particular time or that the prices that you receive when you sell will be favorable.

The underwriters are offering the ordinary shares subject to their acceptance of the ordinary shares from us and subject to prior sale. The underwriters reserve the right to withdraw, cancel or modify offers to the public and to reject orders in whole or in part. In addition, the underwriters have advised us that they do not intend to confirm sales to any account over which they exercise discretionary authority.

### Commission and Expenses

The underwriters have advised us that they propose to offer the ordinary shares to the public at the initial public offering price set forth on the cover page of this prospectus and to certain dealers, which may include the underwriters, at that price less a concession not in excess of \$ \_\_\_\_\_ per ordinary share. The underwriters may allow, and certain dealers may reallocate, a discount from the concession not in excess of \$ \_\_\_\_\_ per ordinary share to certain brokers and dealers. After the offering, the initial public offering price, concession and reallocation to dealers may be reduced by the representatives. No such reduction will change the amount of proceeds to be received by us as set forth on the cover page of this prospectus.

The following table shows the public offering price, the underwriting discounts and commissions that we are to pay the underwriters and the proceeds, before expenses, to us in connection with this offering. Such

amounts are shown assuming both no exercise and full exercise of the underwriters' option to purchase additional ordinary shares.

	PER ORDINARY SHARE		TOTAL	
	WITHOUT OPTION TO PURCHASE ADDITIONAL ORDINARY SHARES	WITH OPTION TO PURCHASE ADDITIONAL ORDINARY SHARES	WITHOUT OPTION TO PURCHASE ADDITIONAL ORDINARY SHARES	WITH OPTION TO PURCHASE ADDITIONAL ORDINARY SHARES
Public offering price	\$	\$	\$	\$
Underwriting discounts and commissions				
Proceeds to us, before expenses				

We estimate expenses payable by us in connection with this offering, other than the underwriting discounts and commissions referred to above, will be approximately \$ . We also have agreed to reimburse the underwriters for up to \$ for their FINRA counsel fee. In accordance with FINRA Rule 5110, this reimbursed fee is deemed underwriting compensation for this offering.

### Determination of Offering Price

Prior to this offering, there has not been a public market for our ordinary shares. Consequently, the initial public offering price for our ordinary shares will be determined by negotiations between us and the representatives. Among the factors to be considered in these negotiations will be prevailing market conditions, our financial information, market valuations of other companies that we and the underwriters believe to be comparable to us, estimates of our business potential, the present state of our development and other factors deemed relevant.

We offer no assurances that the initial public offering price will correspond to the price at which the ordinary shares will trade in the public market subsequent to the offering or that an active trading market for the ordinary shares will develop and continue after the offering.

### Listing

We have applied to list our ordinary shares on The NASDAQ Global Market under the trading symbol "QURE."

### Stamp Taxes

If you purchase ordinary shares offered in this prospectus, you may be required to pay stamp taxes and other charges under the laws and practices of the country of purchase, in addition to the offering price listed on the cover page of this prospectus.

### Option to Purchase Additional Ordinary Shares

We have granted to the underwriters an option, exercisable for 30 days from the date of this prospectus, to purchase, from time to time, in whole or in part, up to an aggregate of ordinary shares from us at the public offering price set forth on the cover page of this prospectus, less underwriting discounts and commissions. If the underwriters exercise this option, each underwriter will be obligated, subject to specified conditions, to purchase a number of additional ordinary shares proportionate to that underwriter's initial purchase commitment as indicated in the table above. This option may be exercised only if the underwriters sell more ordinary shares than the total number set forth on the cover page of this prospectus.

## No Sales of Similar Securities

We, the members of our supervisory board, the members of our management board, our senior management team and holders of all or substantially all our outstanding share capital and other securities have agreed, subject to specified exceptions, not to directly or indirectly:

- sell, offer, contract or grant any option to sell (including any short sale), pledge, transfer, establish an open "put equivalent position" within the meaning of Rule 16a-1(h) under the Securities Exchange Act of 1934, as amended, or
- otherwise dispose of any share capital, options or warrants to acquire share capital, or securities exchangeable or exercisable for or convertible into share capital currently or hereafter owned either of record or beneficially, or
- publicly announce an intention to do any of the foregoing for a period of 180 days after the date of this prospectus without the prior written consent of Jefferies LLC and Leerink Swann LLC.

This restriction terminates after the close of trading of the ordinary shares on and including the 180<sup>th</sup> day after the date of this prospectus.

Jefferies LLC and Leerink Swann LLC may, in their sole discretion and at any time or from time to time before the termination of the 180-day period release all or any portion of the securities subject to lock-up agreements. There are no existing agreements between the underwriters and any of our shareholders who will execute a lock-up agreement, providing consent to the sale of share capital prior to the expiration of the lock-up period.

## Stabilization

The underwriters have advised us that they, pursuant to Regulation M under the Securities Exchange Act of 1934, as amended, certain persons participating in the offering may engage in short sale transactions, stabilizing transactions, syndicate covering transactions or the imposition of penalty bids in connection with this offering. These activities may have the effect of stabilizing or maintaining the market price of the ordinary shares at a level above that which might otherwise prevail in the open market. Establishing short sales positions may involve either "covered" short sales or "naked" short sales.

"Covered" short sales are sales made in an amount not greater than the underwriters' option to purchase additional ordinary shares in this offering. The underwriters may close out any covered short position by either exercising their option to purchase additional ordinary shares or purchasing our ordinary shares in the open market. In determining the source of ordinary shares to close out the covered short position, the underwriters will consider, among other things, the price of ordinary shares available for purchase in the open market as compared to the price at which they may purchase ordinary shares through the option to purchase additional ordinary shares.

"Naked" short sales are sales in excess of the option to purchase additional ordinary shares. The underwriters must close out any naked short position by purchasing ordinary shares in the open market. A naked short position is more likely to be created if the underwriters are concerned that there may be downward pressure on the price of our ordinary shares in the open market after pricing that could adversely affect investors who purchase in this offering.

A stabilizing bid is a bid for the purchase of ordinary shares on behalf of the underwriters for the purpose of fixing or maintaining the price of the ordinary shares. A syndicate covering transaction is the bid for or the purchase of ordinary shares on behalf of the underwriters to reduce a short position incurred by the underwriters in connection with the offering. Similar to other purchase transactions, the underwriter's purchases to cover the syndicate short sales may have the effect of raising or maintaining the market price of our ordinary shares or preventing or retarding a decline in the market price of our ordinary shares. As a result, the price of our ordinary shares may be higher than the price that might otherwise exist in the open market. A penalty bid is an arrangement permitting the underwriters to reclaim the selling concession

otherwise accruing to a syndicate member in connection with the offering if the ordinary shares originally sold by such syndicate member are purchased in a syndicate covering transaction and therefore have not been effectively placed by such syndicate member.

Neither we, nor any of the underwriters make any representation or prediction as to the direction or magnitude of any effect that the transactions described above may have on the price of our ordinary shares. The underwriters are not obligated to engage in these activities and, if commenced, any of the activities may be discontinued at any time.

The underwriters may also engage in passive market making transactions in our ordinary shares on The NASDAQ Global Select Market in accordance with Rule 103 of Regulation M during a period before the commencement of offers or sales of our ordinary shares in this offering and extending through the completion of distribution. A passive market maker must display its bid at a price not in excess of the highest independent bid of that security. However, if all independent bids are lowered below the passive market maker's bid, that bid must then be lowered when specified purchase limits are exceeded.

### **Electronic Distribution**

A prospectus in electronic format may be made available by e-mail or on the web sites or through online services maintained by one or more of the underwriters or their affiliates. In those cases, prospective investors may view offering terms online and may be allowed to place orders online. The underwriters may agree with us to allocate a specific number of ordinary shares for sale to online brokerage account holders. Any such allocation for online distributions will be made by the underwriters on the same basis as other allocations. Other than the prospectus in electronic format, the information on the underwriters' web sites and any information contained in any other web site maintained by any of the underwriters is not part of this prospectus, has not been approved and/or endorsed by us or the underwriters and should not be relied upon by investors.

### **Other Activities and Relationships**

The underwriters and certain of their affiliates are full service financial institutions engaged in various activities, which may include securities trading, commercial and investment banking, financial advisory, investment management, investment research, principal investment, hedging, financing and brokerage activities. The underwriters and certain of their affiliates have, from time to time, performed, and may in the future perform, various commercial and investment banking and financial advisory services for us and our affiliates, for which they received or will receive customary fees and expenses.

In the ordinary course of their various business activities, the underwriters and certain of their affiliates may make or hold a broad array of investments and actively trade debt and equity securities (or related derivative securities) and financial instruments (including bank loans) for their own account and for the accounts of their customers, and such investment and securities activities may involve securities and/or instruments issued by us and our affiliates. If the underwriters or their respective affiliates have a lending relationship with us, they routinely hedge their credit exposure to us consistent with their customary risk management policies. The underwriters and their respective affiliates may hedge such exposure by entering into transactions which consist of either the purchase of credit default swaps or the creation of short positions in our securities or the securities of our affiliates, including potentially the ordinary shares offered hereby. Any such short positions could adversely affect future trading prices of the ordinary shares offered hereby. The underwriters and certain of their respective affiliates may also communicate independent investment recommendations, market color or trading ideas and/or publish or express independent research views in respect of such securities or instruments and may at any time hold, or recommend to clients that they acquire, long and/or short positions in such securities and instruments.

## NOTICE TO INVESTORS

### Australia

This prospectus is not a disclosure document for the purposes of Australia's Corporations Act 2001 (Cth) of Australia, or Corporations Act, has not been lodged with the Australian Securities & Investments Commission and is only directed to the categories of exempt persons set out below. Accordingly, if you receive this prospectus in Australia:

**You confirm and warrant that you are either:**

- a "sophisticated investor" under section 708(8)(a) or (b) of the Corporations Act;
- a "sophisticated investor" under section 708(8)(c) or (d) of the Corporations Act and that you have provided an accountant's certificate to the company which complies with the requirements of section 708(8)(c)(i) or (ii) of the Corporations Act and related regulations before the offer has been made; or
- a "professional investor" within the meaning of section 708(11)(a) or (b) of the Corporations Act.

To the extent that you are unable to confirm or warrant that you are an exempt sophisticated investor or professional investor under the Corporations Act any offer made to you under this prospectus is void and incapable of acceptance.

You warrant and agree that you will not offer any of the shares issued to you pursuant to this prospectus for resale in Australia within 12 months of those securities being issued unless any such resale offer is exempt from the requirement to issue a disclosure document under section 708 of the Corporations Act.

### European Economic Area

In relation to each member state of the European Economic Area which has implemented the Prospectus Directive, each referred to herein as a Relevant Member State, with effect from and including the date on which the Prospectus Directive is implemented in that Relevant Member State, referred to herein as the Relevant Implementation Date, no offer of any securities which are the subject of the offering contemplated by this prospectus has been or will be made to the public in that Relevant Member State other than any offer where a prospectus has been or will be published in relation to such securities that has been approved by the competent authority in that Relevant Member State or, where appropriate, approved in another Relevant Member State and notified to the relevant competent authority in that Relevant Member State in accordance with the Prospectus Directive, except that with effect from and including the Relevant Implementation Date, an offer of such securities may be made to the public in that Relevant Member State:

- to any legal entity which is a "qualified investor" as defined in the Prospectus Directive;
- to fewer than 100 or, if the Relevant Member State has implemented the relevant provision of the 2010 PD Amending Directive, 150, natural or legal persons (other than qualified investors as defined in the Prospectus Directive), as permitted under the Prospectus Directive, subject to obtaining the prior consent of the representatives of the underwriters for any such offer; or
- in any other circumstances falling within Article 3(2) of the Prospectus Directive,

provided that no such offer of securities shall require the Company or any of the underwriters to publish a prospectus pursuant to Article 3 of the Prospectus Directive or supplement a prospectus pursuant to Article 16 of the Prospectus Directive.

For the purposes of this provision, the expression an "offer to the public" in relation to any securities in any Relevant Member State means the communication in any form and by any means of sufficient information on the terms of the offer and the securities to be offered so as to enable an investor to decide to purchase or subscribe the securities, as the same may be varied in that Relevant Member State by any measure implementing the Prospectus Directive in that Relevant Member State and the expression "Prospectus

Directive" means Directive 2003/71/EC (and amendments thereto, including the 2010 PD Amending Directive, to the extent implemented in the Relevant Member State), and includes any relevant implementing measure in the Relevant Member State and the expression "2010 PD Amending Directive" means Directive 2010/73/EU.

## **Hong Kong**

No securities have been offered or sold, and no securities may be offered or sold, in Hong Kong, by means of any document, other than to persons whose ordinary business is to buy or sell shares or debentures, whether as principal or agent; or to "professional investors" as defined in the Securities and Futures Ordinance (Cap. 571) of Hong Kong and any rules made under that Ordinance; or in other circumstances which do not result in the document being a "prospectus" as defined in the Companies Ordinance (Cap. 32) of Hong Kong or which do not constitute an offer to the public within the meaning of the Companies Ordinance (Cap.32) of Hong Kong. No document, invitation or advertisement relating to the securities has been issued or may be issued or may be in the possession of any person for the purpose of issue (in each case whether in Hong Kong or elsewhere), which is directed at, or the contents of which are likely to be accessed or read by, the public of Hong Kong (except if permitted under the securities laws of Hong Kong) other than with respect to securities which are or are intended to be disposed of only to persons outside Hong Kong or only to "professional investors" as defined in the Securities and Futures Ordinance (Cap. 571) of Hong Kong and any rules made under that Ordinance.

This prospectus has not been registered with the Registrar of Companies in Hong Kong. Accordingly, this prospectus may not be issued, circulated or distributed in Hong Kong, and the securities may not be offered for subscription to members of the public in Hong Kong. Each person acquiring the securities will be required, and is deemed by the acquisition of the securities, to confirm that he is aware of the restriction on offers of the securities described in this prospectus and the relevant offering documents and that he is not acquiring, and has not been offered any securities in circumstances that contravene any such restrictions.

## **Japan**

The offering has not been and will not be registered under the Financial Instruments and Exchange Law of Japan (Law No. 25 of 1948 of Japan, as amended), or FIEL, and the Initial Purchaser will not offer or sell any securities, directly or indirectly, in Japan or to, or for the benefit of, any resident of Japan (which term as used herein means, unless otherwise provided herein, any person resident in Japan, including any corporation or other entity organized under the laws of Japan), or to others for re-offering or resale, directly or indirectly, in Japan or to a resident of Japan, except pursuant to an exemption from the registration requirements of, and otherwise in compliance with, the FIEL and any other applicable laws, regulations and ministerial guidelines of Japan.

## **Singapore**

This prospectus has not been and will not be lodged or registered with the Monetary Authority of Singapore. Accordingly, this prospectus and any other document or material in connection with the offer or sale, or the invitation for subscription or purchase of the securities may not be issued, circulated or distributed, nor may the securities be offered or sold, or be made the subject of an invitation for subscription or purchase, whether directly or indirectly, to the public or any member of the public in Singapore other than (i) to an institutional investor under Section 274 of the Securities and Futures Act, Chapter 289 of Singapore, or the SFA, (ii) to a relevant person as defined under Section 275(2), or any person pursuant to Section 275(1A) of the SFA, and in accordance with the conditions, specified in Section 275 of the SFA, or (iii) otherwise pursuant to, and in accordance with the conditions of any other applicable provision of the SFA.



Where the securities are subscribed or purchased under Section 275 of the SFA by a relevant person which is:

- a corporation (which is not an accredited investor as defined under Section 4A of the SFA) the sole business of which is to hold investments and the entire share capital of which is owned by one or more individuals, each of whom is an accredited investor; or
- a trust (where the trustee is not an accredited investor) whose sole purpose is to hold investments and each beneficiary is an accredited investor,

shares, debentures and units of shares and debentures of that corporation or the beneficiaries' rights and interest in that trust shall not be transferable for six months after that corporation or that trust has acquired the Offer Shares under Section 275 of the SFA except:

- to an institutional investor under Section 274 of the SFA or to a relevant person defined in Section 275(2) of the SFA, or to any person pursuant to an offer that is made on terms that such shares, debentures and units of shares and debentures of that corporation or such rights and interest in that trust are acquired at a consideration of not less than \$200,000 (or its equivalent in a foreign currency) for each transaction, whether such amount is to be paid for in cash or by exchange of securities or other assets, and further for corporations, in accordance with the conditions, specified in Section 275 of the SFA;
- where no consideration is given for the transfer; or

where the transfer is by operation of law.

## Switzerland

The securities may not be publicly offered in Switzerland and will not be listed on the SIX Swiss Exchange, or SIX, or on any other stock exchange or regulated trading facility in Switzerland. This prospectus has been prepared without regard to the disclosure standards for issuance prospectuses under art. 652a or art. 1156 of the Swiss Code of Obligations or the disclosure standards for listing prospectuses under art. 27 ff. of the SIX Listing Rules or the listing rules of any other stock exchange or regulated trading facility in Switzerland. Neither this prospectus nor any other offering or marketing material relating to the securities or the offering may be publicly distributed or otherwise made publicly available in Switzerland.

Neither this prospectus nor any other offering or marketing material relating to the offering, the Company or the securities have been or will be filed with or approved by any Swiss regulatory authority. In particular, this prospectus will not be filed with, and the offer of securities will not be supervised by, the Swiss Financial Market Supervisory Authority FINMA, or FINMA, and the offer of securities has not been and will not be authorized under the Swiss Federal Act on Collective Investment Schemes, or CISA. The investor protection afforded to acquirers of interests in collective investment schemes under the CISA does not extend to acquirers of securities.

## United Kingdom

This prospectus is only being distributed to, and is only directed at, persons in the United Kingdom that are qualified investors within the meaning of Article 2(1)(e) of the Prospectus Directive that are also (i) investment professionals falling within Article 19(5) of the Financial Services and Markets Act 2000 (Financial Promotion) Order 2005, as amended, referred to herein as the Order, and/or (ii) high net worth entities falling within Article 49(2)(a) to (d) of the Order and other persons to whom it may lawfully be communicated. Each such person is referred to herein as a Relevant Person.

This prospectus and its contents are confidential and should not be distributed, published or reproduced (in whole or in part) or disclosed by recipients to any other persons in the United Kingdom. Any person in the United Kingdom that is not a Relevant Person should not act or rely on this document or any of its contents.

## EXPENSES OF THE OFFERING

We estimate that our expenses in connection with this offering, other than underwriting discounts and commissions, will be as follows:

EXPENSES	AMOUNT
U.S. Securities and Exchange Commission registration fee	\$
FINRA filing fee	\$
Nasdaq listing fee	\$
Printing and engraving expenses	\$
Legal fees and expenses	\$
Accounting fees and expenses	\$
Miscellaneous costs	\$
Total	\$

All amounts in the table are estimates except the U.S. Securities and Exchange Commission registration fee and the FINRA filing fee. We will pay all of the expenses of this offering.

## LEGAL MATTERS

Legal matters with respect to U.S. federal and New York law in connection with this offering will be passed upon for us by Wilmer Cutler Pickering Hale and Dorr LLP, London, England. Certain legal matters with respect to Dutch law in connection with the validity of the shares being offered by this prospectus and other legal matters will be passed upon for us by Rutgers Posch Visée Endedijk N.V., Amsterdam, the Netherlands. Wilmer Cutler Pickering Hale and Dorr LLP, Boston, Massachusetts, has provided an opinion as to matters of U.S. federal income tax. Van Campen Liem (Liem&Partners N.V.), Amsterdam, the Netherlands has provided an opinion as to matters of Dutch income tax. Covington & Burling LLP, New York, New York is U.S. federal and New York law counsel for the underwriters in connection with this offering. Nauta Dutilh N.V., Amsterdam, the Netherlands is counsel to the underwriters with respect to Dutch law.

## EXPERTS

The consolidated financial statements of uniQure B.V. as of December 31, 2010, 2011 and 2012, and for each of the two years in the period ended December 31, 2012, included in this prospectus have been so included in reliance on the report of PricewaterhouseCoopers Accountants N.V., an independent registered public accounting firm, given on the authority of said firm as experts in auditing and accounting.

The current address of PricewaterhouseCoopers Accountants N.V. is Newtonlaan 205, 3584 BH Utrecht, the Netherlands.

## ENFORCEABILITY OF CIVIL LIABILITIES

uniQure N.V. is incorporated under the laws of the Netherlands. Substantially all of our business is conducted, and substantially all of our assets are located, in the Netherlands. Most of our directors and the experts named in this prospectus are residents of, and most of their assets are located in, jurisdictions outside the United States. As a result, it may be difficult for you to serve process on us or these persons within the United States or to enforce against us or these persons in courts in the United States, judgments of these courts predicated upon the civil liability provisions of U.S. securities laws. In addition, it is not clear whether a Dutch court would impose civil liability on us, members of our board or any of the experts named in this prospectus in an original action based solely upon the federal securities laws of the United States brought in a court of competent jurisdiction in the Netherlands.

As there is no treaty on the reciprocal recognition and enforcement of judgments in civil and commercial matters between the United States and the Netherlands, courts in the Netherlands will not automatically recognize and enforce a final judgment rendered by a U.S. court. In order to obtain a judgment enforceable in the Netherlands, claimants must litigate the relevant claim again before a Dutch court of competent jurisdiction. Under current practice, however, a Dutch court will generally uphold and consider as conclusive evidence a final and conclusive judgment for the payment of money rendered by a U.S. court and not rendered by default, provided that the Dutch court finds that:

- the jurisdiction of the United States court has been based on grounds that are internationally acceptable;
- the final judgment results from proceedings compatible with Dutch concepts of due process;
- the final judgment does not contravene public policy of the Netherlands; and
- the final judgment has not been rendered in proceedings of a penal, revenue or other public law nature.

If a Dutch court upholds and regards as conclusive evidence the final judgment, that court generally will grant the same judgment without litigating again on the merits.

In the event a third party is liable to a Dutch company, only the company itself can bring a civil action against that party. The individual shareholders do not have the right to bring an action on behalf of the company. Only in the event that the cause for the liability of a third party to the company also constitutes a tortious act directly against a shareholder does that shareholder have an individual right of action against such third party in its own name. The Dutch Civil Code does provide for the possibility to initiate such actions collectively. A foundation or an association whose objective is to protect the rights of a group of persons having similar interests can institute a collective action. The collective action itself cannot result in an order for payment of monetary damages but may only result in a declaratory judgment (*verklaring voor recht*). In order to obtain compensation for damages, the foundation or association and the defendant may reach—often on the basis of such declaratory judgment—a settlement. A Dutch court may declare the settlement agreement binding upon all the injured parties with an opt-out choice for an individual injured party. An individual injured party may also itself institute a civil claim for damages.

## WHERE YOU CAN FIND ADDITIONAL INFORMATION

We have filed with the SEC a registration statement on Form F-1 under the Securities Act, including relevant exhibits and schedules, with respect to the ordinary shares to be sold in this offering. This prospectus, which constitutes a part of the registration statement, does not contain all of the information contained in the registration statement. You should read the registration statement and its exhibits for further information with respect to us and our shares. Some of these exhibits consist of documents or contracts that are described in this prospectus in summary form. You should read the entire document or contract for the complete terms. You may read and copy the registration statement and its exhibits at the SEC's Public Reference Room at 100 F Street N.E., Room 1580, Washington, D.C. 20549. You may obtain information on the operation of the Public Reference Room by calling the SEC at 1-800-SEC-0330. In addition, the SEC maintains an internet website at [www.sec.gov](http://www.sec.gov), from which you can electronically access the registration statement and its exhibits.

After this offering, we will be subject to the reporting requirements of the Securities Exchange Act of 1934, or Exchange Act, applicable to foreign private issuers. Because we are a foreign private issuer, the SEC's rules do not require us to deliver proxy statements pursuant to Section 14 of the Exchange Act or to file quarterly reports on Form 10-Q, among other things. However, we plan to produce quarterly financial reports and furnish them to the SEC not later than 45 days after the end of each of the first three quarters of our fiscal year and to file our annual report on Form 20-F not later than 90 days after the end of our fiscal year. In addition, our "insiders" are not subject to the SEC's rules that prohibit short-swing trading. Our annual consolidated financial statements will be prepared in accordance with International Financial Reporting Standards as issued by the International Accounting Standards Board and certified by an independent public accounting firm.

We also maintain an internet website at [www.unique.com](http://www.unique.com). Information contained in or connected to our website is not a part of this prospectus.

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UNIQUE B.V.

Unaudited Condensed Consolidated Balance Sheets  
(€ in thousands)

	NOTE	DECEMBER 31, 2012	SEPTEMBER 30, 2013
<b>Assets</b>			
<b>Non-current assets</b>			
Intangible assets	8	3,278	6,770
Property, plant and equipment	7	1,185	1,353
Other non-current assets	9	—	917
<b>Total non-current assets</b>		<u>4,463</u>	<u>9,040</u>
<b>Current assets</b>			
Receivables from related parties	10,22	26	726
Trade and Other Receivables	10	815	2,051
Inventories	11	—	427
Cash and cash equivalents	12	263	31,427
<b>Total current assets</b>		<u>1,104</u>	<u>34,631</u>
<b>Total assets</b>		<u>5,567</u>	<u>43,671</u>
<b>Equity</b>			
Share capital		483	609
Share premium		114,795	142,444
Other reserves		1,508	5,924
Accumulated deficit		(117,234)	(137,656)
<b>Total equity</b>	13	<u>(448)</u>	<u>11,321</u>
<b>Liabilities</b>			
<b>Non-current liabilities</b>			
Borrowings	15	—	7,291
Financial lease liabilities	15	450	342
Deferred revenue	16	—	15,899
<b>Total non-current liabilities</b>		<u>450</u>	<u>23,532</u>
<b>Current liabilities</b>			
Trade and other payables	14	4,067	6,574
Debt to related party—financial liability	15	1,366	—
Debt to related party—embedded derivative	15	132	892
Borrowings—embedded derivative	15	—	273
Deferred revenue	16	—	1,079
<b>Total Current Liabilities</b>		<u>5,565</u>	<u>8,818</u>
<b>Total liabilities</b>		<u>6,015</u>	<u>32,350</u>
<b>Total equity and liabilities</b>		<u>5,567</u>	<u>43,671</u>

The notes are an integral part of these condensed consolidated financial statements.

UNIQUE B.V.

**Unaudited Condensed Consolidated Statements of Comprehensive Income**  
(€ in thousands, except share and per share data)

	NOTE	NINE MONTHS ENDED SEPTEMBER 30,	
		2012	2013
License revenues	16	—	220
Collaboration revenues	16	—	1,831
<b>Total revenues</b>		<b>—</b>	<b>2,051</b>
Cost of goods sold	22	—	(800)
<b>Gross profit</b>		<b>—</b>	<b>1,251</b>
Other income		315	686
Research and development expenses	17	(5,690)	(9,856)
Selling, general and administrative expenses	18	(4,438)	(7,612)
Other losses—net		(82)	(269)
<b>Total Operating Costs</b>		<b>(9,895)</b>	<b>(17,051)</b>
<b>Operating result</b>		<b>(9,895)</b>	<b>(15,800)</b>
Finance income		16	48
Finance expense	15	(545)	(4,676)
<b>Finance income/(expense)—net</b>		<b>(529)</b>	<b>(4,628)</b>
<b>Result before corporate income taxes</b>		<b>(10,424)</b>	<b>(20,428)</b>
<b>Corporate income taxes</b>		<b>—</b>	<b>—</b>
<b>Net Loss</b>		<b>(10,424)</b>	<b>(20,428)</b>
Other comprehensive income	19	—	6
<b>Total comprehensive loss*</b>		<b>(10,424)</b>	<b>(20,422)</b>
Loss per share attributable to the equity holders of the Company during the year			
<b>Basic and diluted loss per share</b>	21	<b>(0.25)</b>	<b>(0.39)</b>

\* Total comprehensive loss is fully attributable to equity holders of the group

The notes are an integral part of these condensed consolidated financial statements.

UNIQUE B.V.

Unaudited Condensed Consolidated Statement of Changes in Equity  
(€ in thousands)

	TOTAL SHARE CAPITAL	SHARE PREMIUM	OTHER RESERVES	ACCUMULATED DEFICIT	TOTAL EQUITY
<b>Balance at January 1, 2012</b>	237	99,947	2,728	(105,505)	<b>(2,593)</b>
Result for the period				(10,424)	<b>(10,424)</b>
Capital contributions	241	14,579			<b>14,820</b>
Share based payment/expense			1,228		<b>1,228</b>
Adjustment to reserves on expiration of the AMT option scheme			(2,987)	2,987	<b>—</b>
<b>Balance at September 30, 2012</b>	<b>478</b>	<b>114,526</b>	<b>969</b>	<b>(112,942)</b>	<b>3,031</b>
Result for the period				(4,292)	<b>(4,292)</b>
Capital contributions	5	269			<b>274</b>
Share-based payment/expense			539		<b>539</b>
<b>Balance at December 31, 2012</b>	<b>483</b>	<b>114,795</b>	<b>1,508</b>	<b>(117,234)</b>	<b>(448)</b>
Result for the period				(20,428)	<b>(20,428)</b>
Other Comprehensive Income				6	<b>6</b>
Capital contributions	126	27,649			<b>27,775</b>
Result on conversion of loan			3,005		<b>3,005</b>
Share-based payment/expense			1,411		<b>1,411</b>
<b>Balance at September 30, 2013</b>	<b>609</b>	<b>142,444</b>	<b>5,924</b>	<b>(137,656)</b>	<b>11,321</b>

The notes are an integral part of these condensed consolidated financial statements.



UNIQUE B.V.

Unaudited Condensed Consolidated Statement of Cash Flows  
(€ in thousands)

		NINE MONTHS ENDED SEPTEMBER 30,	
	NOTE	2012	2013
<b>Cash flow from operating activities</b>			
Result before corporate income tax		(10,424)	(20,428)
Adjustments for:			
—Depreciation	7	403	398
—Derivative result	12	—	2,339
—Derivative result arising on early conversion of a loan	12	464	1,333
—Exchange result		82	269
—Share-based payment expenses	20	1,228	1,411
—Changes in other non-current assets		—	(917)
—Changes in trade and other receivables		228	(1,782)
—Movement in inventories	11	—	(427)
—Changes in trade and other payables	13	(853)	(141)
—Changes in deferred revenue and provisions		—	16,978
—Movement in other liabilities		235	1,702
—Interest (income)/expense		66	956
Cash used in operations		(8,571)	1,691
Net interest paid		(8)	(17)
<b>Net cash used in operating activities</b>		<b>(8,579)</b>	<b>1,674</b>
<b>Cash flow from investing activities</b>			
Purchases of property, plant and equipment	7,12	(149)	(536)
Purchases of intangible assets	8	(386)	3,647
Interest received		108	4
<b>Net cash used in investing activities</b>		<b>(427)</b>	<b>4,179</b>
<b>Cash flow from financing activities</b>			
Capital contribution from shareholders	12,13	9,500	14,278
Convertible loans drawn down	12,15	—	11,999
Proceeds from borrowings	15	—	7,492
Redemption of financial lease	15	(67)	(106)
Repayments of borrowings	15	—	—
<b>Net cash generated from financing activities</b>		<b>9,433</b>	<b>33,663</b>
<b>Net increase in cash, cash equivalents, and other bank overdrafts</b>		<b>427</b>	<b>31,158</b>
Currency effect cash and cash equivalents		—	6
Cash, cash equivalents, and other bank overdrafts at beginning of the period		1,100	263
<b>Cash, cash equivalents, and other bank overdrafts cash at end of the period</b>	12	<b>1,527</b>	<b>31,427</b>

The notes are an integral part of these condensed consolidated financial statements.

**UNIQUIRE B.V.****Notes to Unaudited Condensed Consolidated Financial Statements****1. General information****uniQure B.V.**

uniQure B.V. ("uniQure" or the "Company") is a biopharmaceutical company domiciled in The Netherlands with headquarters at Meibergdreef 61, 1105 BA, Amsterdam, The Netherlands.

The Company is a leader in the field of gene therapy, and has developed the first product to receive regulatory approval in the European Union and as well as multiple collaborations designed to accelerate the development of a broad pipeline of additional product candidates. The Company was incorporated in January 2012 to acquire and continue the gene therapy business ("AMT Business") of Amsterdam Molecular Therapeutics (AMT) Holding N.V. ("AMT") and its subsidiaries (collectively, the "AMT Group") and to facilitate additional financing, as described further below. The acquisition by uniQure of the AMT Business was announced on February 17, 2012 and completed on April 5, 2012 (uniQure did not acquire AMT, and following the sale of the AMT business to uniQure, AMT was put into liquidation). The acquisition by uniQure of the AMT Business is accounted for as a reverse acquisition, and accordingly the financial statements for the AMT Business, including its trading history, are incorporated into the financial statements of the Company and presented as a continuous trading history. Further details are set out in Note 1 to the audited consolidated financial statements for the year ended December 31, 2012.

As used in these condensed consolidated interim financial statements, unless the context indicates otherwise, all references to "uniQure" or the "Company" refer to uniQure and its consolidated subsidiaries.

**Organizational structure of the uniQure Group**

uniQure B.V. is the ultimate parent of the following group of entities which were transferred to uniQure's ownership as part of the transaction with AMT (as described above) and which were renamed following the transaction, as follows:

<i>Company name</i>	<i>Formerly known as</i>
uniQure biopharma B.V.	Amsterdam Molecular Therapeutics (AMT) B.V.
uniQure IP B.V.	Amsterdam Molecular Therapeutics (AMT) IP B.V.
uniQure Manufacturing B.V.	AMT manufacturing B.V.
uniQure Assay Development B.V.	AMT Assay Development B.V.
uniQure Research B.V.	AMT Research B.V.
uniQure non clinical B.V.	AMT non clinical B.V.
uniQure QA B.V.	AMT QA B.V.
uniQure Process Development B.V.	AMT Process Development B.V.
uniQure clinical B.V.	AMT clinical B.V.
Stichting participatie AMT <sup>(1)</sup>	Stichting participatie AMT <sup>(1)</sup>
uniQure Inc. <sup>(2)</sup>	

<sup>(1)</sup> Stichting participatie AMT is a Trust, not a company, but met the conditions for consolidation within uniQure's consolidated financial statements. Stichting participatie AMT was established to facilitate AMT's employee incentive schemes for the period up to 2010.

<sup>(2)</sup> In May 2013 the Company incorporated uniQure Inc., a Delaware corporation and wholly owned subsidiary of uniQure biopharma B.V.

**UNIQUE B.V.**

**Notes to Unaudited Condensed Consolidated Financial Statements**

**Significant shareholders**

The Company's significant shareholders at the date of publication of these interim statements are:

Advent Venture Partners  
Coller Capital  
Chiesi Farmaceutici S.p.A  
Forbion Capital Partners  
Gilde Healthcare Partners  
Grupo Netco and affiliates  
Lupus Alpha PE Champions  
Omnes Capital (formerly Credit Agricole Private Equity)

**2. Summary of Significant Accounting Policies**

**2.1 Basis of Preparation**

These unaudited condensed consolidated financial statements of the Company were prepared in accordance with International Accounting Standard 34, "Interim Financial Reporting". Certain information and disclosures normally included in consolidated financial statements prepared in accordance with IFRS have been condensed or omitted. Accordingly, these condensed consolidated financial statements should be read in conjunction with the Company's annual consolidated financial statements for the year ended December 31, 2012 which have been prepared in accordance with IFRS as issued by the International Accounting Standards Board and as adopted by the European Union.

The preparation of financial statements in conformity with IFRS requires the use of certain critical accounting estimates. It also requires management to exercise its judgment in the process of applying the Company's accounting policies. The areas involving a higher degree of judgment or complexity, or areas where assumptions and estimates are significant to these condensed consolidated financials are disclosed in Note 4.

During the nine months ended September 30, 2013, the Company identified certain adjustments that impact the six months ended June 30, 2013. These adjustments related to an increase in revenues totaling €283,000, a decrease in share based compensation expenses totaling €312,000, and an increase in finance expenses totaling €203,000, which were recognized during the nine months ended September 30, 2013, but which should have already been recognized during the six months ended June 30, 2013. The net impact to the unaudited condensed consolidated statements of Comprehensive income of these adjustments totals €392,000, and the Company will restate the results for the six months ended June 30, 2013 for comparative purposes, when next presented.

**2.2 Changes in Accounting Policy and Disclosures**

The accounting policies adopted are consistent with those of the previous financial year, except as described below.

# UNIQURE B.V.

## Notes to Unaudited Condensed Consolidated Financial Statements

### a) New and amended standards adopted by the Company

The following standards and amendments to standards became effective for annual periods on January 1, 2013 and have been adopted by the Company in the preparation of the condensed consolidated financial statements:

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IFRS 10	Consolidated Financial Statements
IFRS 11	Joint Arrangements
IFRS 12	Disclosures of Interest in Other Entities
IFRS 13	Fair Value Measurement
IAS 19	Employee Benefits
IFRIC 21 <sup>(1)</sup>	Levies

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<sup>(1)</sup> Applicable for accounting periods beginning on or after January 1, 2014, however uniQure has adopted this standard early.

- IFRS 10, "Consolidated financial statements", builds on existing principles by identifying the concept of control as the determining factor in whether an entity should be included within the consolidated financial statements of the parent company. The standard provides additional guidance to assist in the determination of control where this is difficult to assess. The Company has assessed IFRS 10's full impact and adopted IFRS 10 on January 1, 2013. This standard does not have a material impact on the Company.
- IFRS 11, "Joint arrangements", outlines the accounting by entities that jointly control an arrangement. Joint control involves the contractual agreed sharing of control and arrangements subject to joint control are classified as either a joint venture (representing a share of net assets and equity accounted) or a joint operation (representing rights to assets and obligations for liabilities, accounted for accordingly). The Company has assessed IFRS 11's full impact and adopted IFRS 11 on January 1, 2013. This standard does not have a material impact on the Company.
- IFRS 12, "Disclosures of Interests in Other Entities", includes the disclosure requirements for all forms of interests in other entities, including joint arrangements, associates, special purpose vehicles and other off balance sheet vehicles. The Company has assessed IFRS 12's full impact and adopted IFRS 12 on January 1, 2013. This standard does not have a material impact on the Company.
- IFRS 13, "Fair value measurement," aims to improve consistency and reduce complexity by providing a precise definition of fair value and a single source of fair value measurement and disclosure requirements for use across IFRSs. The requirements, which are largely aligned between IFRSs and US GAAP, do not extend the use of fair value accounting but provide guidance on how it should be applied where its use is already required or permitted by other standards within IFRSs or US GAAP. The Company has assessed IFRS 13's full impact and adopted IFRS 13 on January 1, 2013. This standard does not have a material impact on the Company.
- IAS 19, "Employee benefits", was amended in June 2011. The amendments outline the accounting requirements for employee benefits, including short- term benefits (e.g. wages and salaries, annual leave), post-employment benefits such as retirement benefits, other long-term benefits (e.g. long service leave) and termination benefits. The Company only participates in defined contribution plans; therefore, this amendment does not impact the Company.
- IFRIC 21, "Levies" was issued in May 2013. It addresses when an entity should recognize a liability to pay a government levy (other than income taxes). IFRIC 21 is an interpretation of IAS 37, Provisions, Contingent Liabilities and Contingent Assets. The interpretation is applicable for annual periods beginning on or after January 1, 2014. Early application is permitted. The Company has assessed IFRIC 21's full impact and adopted IFRIC 21 on January 1, 2013. This standard will not have a material impact on the Company.

**UNIQURE B.V.****Notes to Unaudited Condensed Consolidated Financial Statements**

The adoption of these new standards and amendments did not materially impact the Company's financial position or results of operations.

***b) New and amended standards not yet adopted by the Company***

There are no standards which are currently available for early adoption which are expected to have a significant effect on the condensed consolidated financial statements of the Company.

**2.3 Consolidation**

Subsidiaries are all entities over which the Company has the power to govern the financial and operating policies generally accompanying a shareholding of more than 50% of the voting rights. The existence and effect of potential voting rights that are currently exercisable or convertible are considered when assessing whether the Company controls another entity. Subsidiaries are fully consolidated from the date on which control is transferred to the Company. Subsidiaries are de-consolidated from the date that control ceases.

Inter-company transactions, balances, income and expenses on transactions between group companies are eliminated. Profits and losses resulting from inter-company transactions that are recognized in assets are also eliminated. Accounting policies of subsidiaries have been changed where necessary to ensure consistency with the policies adopted by the Company.

**2.4 Inventories**

Inventories are stated at the lower of cost and net realizable value. Cost is determined using the first-in, first-out (FIFO) method. The cost of finished goods and work in progress comprises design costs, raw materials, direct labor, other direct costs and related production overheads (based on normal operating capacity). It excludes borrowing costs. Net realizable value is the estimated selling price in the ordinary course of business, less applicable variable selling expenses.

**2.5 Development costs**

Development costs are capitalized once the conditions set out in IAS 38 are satisfied.

On March 21, 2013, the Company secured a €10.0 million convertible loan, which provided resources to complete development of Glybera. Accordingly, from March 21, 2013, the Company has capitalized development costs relating to Glybera. Following commercial launch of the product by uniQure's commercial partner, Chiesi, which is expected to occur in the first half of 2014, the intangible asset will be carried at its cost less any accumulated amortization and any accumulated impairment losses (cost model). The estimated useful life over which the intangible will be amortized is estimated at approximately 19 years.

**2.6 Revenues and other income**

Revenues comprise the fair value of the consideration received or receivable for the sale of licenses and services in the ordinary course of the Company's activities. Revenues are shown net of value-added tax, returns, rebates and discounts and after eliminating sales within the group.

**License revenues**

License revenues consist of upfront payments and milestone payments.

***a) Upfront payments***

Revenues from non-refundable, up-front payments are initially reported as deferred revenue on the consolidated balance sheet and are recognized in income as earned over the period of the development, commercialization, collaboration or the manufacturing obligation.

**UNIQUIRE B.V.****Notes to Unaudited Condensed Consolidated Financial Statements*****b) Milestone payments***

Sales related milestone payments will be recognized in full in the period in which the relevant milestone is achieved.

**Collaboration revenues**

Collaboration revenues consist of revenues generated from collaborative research and development arrangements. Such agreements may consist of multiple elements and provide for varying consideration terms, such as up-front, milestone and similar payments which require significant analysis in order to determine the appropriate method of revenue recognition.

Where such arrangements can be divided into separate units of accounting (each unit constituting a separate earnings process), the arrangement consideration is allocated to the different units based on their relative fair values and recognized over the respective performance period. Where the arrangement cannot be divided into separate units, the individual deliverables are combined as a single unit of accounting and the total arrangement consideration is recognized over the estimated collaboration period.

**2.7 Other income**

uniQure's other income comprises certain subsidies that support uniQure's research efforts in defined research and development projects. These subsidies generally provide for reimbursement of approved costs incurred as defined in various grants.

**2.8 Foreign currency translation*****a) Functional and presentation currency***

Items included in the financial statements of each of uniQure's entities are measured using the currency of the primary economic environment in which the entity operates ("the functional currency") which historically in all cases has been Euros. The consolidated financial statements are presented in Euros, which is the Company's functional and presentation currency.

***b) Transactions and balances***

Foreign currency transactions are translated into the functional currency using the exchange rates prevailing at the dates of the transactions. Foreign exchange gains and losses resulting from the settlement of such transactions and from the translation at year-end exchange rates of monetary assets and liabilities denominated in foreign currencies are recognized in the income statement. Foreign exchange gains and losses that relate to borrowings and cash and cash equivalents are presented within "Finance income" or "Finance expenses" while all other foreign exchange gains and losses are presented within "Other losses—net" on the Consolidated Statement of Comprehensive Income.

**3. Financial risk management****3.1 Financial risk factors**

The Company's activities expose it to a variety of financial risks: market risk (including currency risk, fair value interest rate risk, cash flow interest rate risk and price risk), credit risk and liquidity risk.

The condensed consolidated financial statements do not include all financial risk management information and disclosures required in the annual consolidated financial statements, and should be read in conjunction with the Company's annual consolidated financial statements for the period ended December 31, 2012.

There have been no changes in the Company's finance department, which is responsible for financial risk management, or in the Company's financial risk management policies, since December 31, 2012.

# UNIQUE B.V.

## Notes to Unaudited Condensed Consolidated Financial Statements

The table below analyzes the Company's financial liabilities in relevant maturity groupings based on the length of time until the contractual maturity date, as at the balance sheet date. The amounts disclosed in the table are the contractual undiscounted cash flows. Balances due within 12 months equal their carrying value balances as the impact of discounting is not significant.

	LESS THAN 1 YEAR	BETWEEN 1 AND 2 YEARS	BETWEEN 2 AND 5 YEARS	OVER 5 YEARS
	(€ in thousands)			
<b>At December 31, 2012</b>				
Borrowings (excl. finance lease liabilities)	—	—	—	—
Financial lease liabilities	151	450	—	—
Debt to related party	1,618	—	—	—
Trade and other payables	3,916	—	—	—
<b>Total</b>	<b>5,685</b>	<b>450</b>	<b>—</b>	<b>—</b>
<b>At September 30, 2013</b>				
Borrowings (excl. finance lease liabilities)	1,165	2,690	4,601	—
Financial lease liabilities	153	165	177	—
Debt to related party	—	—	—	—
Trade and other payables	6,421	—	—	—
<b>Total</b>	<b>7,739</b>	<b>2,855</b>	<b>4,778</b>	<b>—</b>

The Financial instruments by category are as follows:

	FOR PERIOD ENDED DECEMBER 31, 2012				
	LOANS AND RECEIVABLES	ASSETS AT FAIR VALUE THROUGH PROFIT AND LOSS	DERIVATIVES USED FOR HEDGING	AVAILABLE FOR SALE	TOTAL
	(€ in thousands)				
<b>Assets as per balance sheet</b>					
Receivables from related parties	26	—	—	—	26
Trade and other receivables	815	—	—	—	815
Cash and cash equivalents	263	—	—	—	263
<b>Total</b>	<b>1,104</b>	<b>—</b>	<b>—</b>	<b>—</b>	<b>1,104</b>

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Notes to Unaudited Condensed Consolidated Financial Statements

	LIABILITIES AT FAIR VALUE THROUGH PROFIT AND LOSS	DERIVATIVES USED FOR HEDGING	OTHER FINANCIAL LIABILITIES AT AMORTIZED COST	TOTAL
	(€ in thousands)			
<b>Liabilities as per balance sheet</b>				
Debt to related party	132	—	1,366	1,498
Financial lease liabilities	—	—	601	601
Trade and other payables	—	—	3,916	3,916
<b>Total</b>	<b>132</b>	<b>—</b>	<b>5,883</b>	<b>6,015</b>

	FOR PERIOD ENDED SEPTEMBER 30, 2013				
	LOANS AND RECEIVABLES	ASSETS AT FAIR VALUE THROUGH PROFIT AND LOSS	DERIVATIVES USED FOR HEDGING	AVAILABLE FOR SALE	TOTAL
	(€ in thousands)				
<b>Assets as per balance sheet</b>					
Receivables from related parties	726	—	—	—	726
Trade and other receivables	2,051	—	—	—	2,051
Cash and cash equivalents	31,427	—	—	—	31,427
<b>Total</b>	<b>34,204</b>	<b>—</b>	<b>—</b>	<b>—</b>	<b>34,204</b>

	LIABILITIES AT FAIR VALUE THROUGH PROFIT AND LOSS	DERIVATIVES USED FOR HEDGING	OTHER FINANCIAL LIABILITIES AT AMORTIZED COST	TOTAL
	(€ in thousands)			
<b>Liabilities as per balance sheet</b>				
Borrowings (excl. finance lease liabilities)	273	—	7,291	7,564
Debt to related party	892	—	—	892
Finance lease liabilities	—	—	495	495
Trade and other payables excluding non-financial liabilities	—	—	6,574	6,574
<b>Total</b>	<b>1,165</b>	<b>—</b>	<b>14,360</b>	<b>15,525</b>

For financial instruments that are measured on the balance sheet at fair value, IFRS 7 requires disclosure of fair value measurements by level of the following fair value measurement hierarchy:

- Quoted prices (unadjusted) in active markets for identical assets or liabilities (level 1);
- Inputs other than quoted prices included within level 1 that are observable for the asset or liability, either directly (that is, as prices) or indirectly (that is, derived from prices) (level 2); and
- Inputs for the asset or liability that are not based on observable market data (that is, unobservable inputs) (level 3).



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### Notes to Unaudited Condensed Consolidated Financial Statements

The fair value of financial instruments that are not traded in an active market (for example, over-the-counter derivatives) is determined by using valuation techniques. These valuation techniques maximize the use of observable market data where it is available and rely as little as possible on entity specific estimates. If all significant inputs required to ascertain the fair value of an instrument are observable, the instrument is included in level 2.

If one or more of the significant inputs is not based on observable market data, the instrument is included in level 3.

The carrying amount of a financial asset or financial liability is a reasonable approximation of the fair value and therefore information about the fair values of each class has not been disclosed.

	LEVEL 1	LEVEL 2	LEVEL 3	TOTAL
<b>At December 31, 2012</b>				
Debt to related party—embedded derivative (warrants)	—	—	132	<b>132</b>
Borrowings—embedded derivative (warrants)	—	—	—	<b>—</b>
	<u>—</u>	<u>—</u>	<u>132</u>	<u><b>132</b></u>

	LEVEL 1	LEVEL 2	LEVEL 3	TOTAL
<b>At September 30, 2013</b>				
Debt to related party—embedded derivative (warrants)	—	—	892	<b>892</b>
Borrowings—embedded derivative (warrants)	—	—	273	<b>273</b>
	<u>—</u>	<u>—</u>	<u>1,165</u>	<u><b>1,165</b></u>

	LEVEL 3
Opening Balance at January 1, 2013	132
Transfers to/(from) level 3	366
Movement in Equity on early conversion of the convertible loan	(3,005)
Losses recognized in Profit and Loss during the nine months ended September 30, 2013	3,672
Closing balance at September 30, 2013	<b>1,165</b>
Total losses for the period included in P&L for assets held at the end of the reporting period, under Finance expenses	3,672

#### Group valuation processes

The fair value of the level 3 liabilities as of September 30, 2013 have been determined using a Black-Scholes option pricing model. Key inputs include the risk-free rate, volatility, term, exercise price, and fair value of ordinary shares. The values are included within the tables presented above. Changes in the fair values are analyzed at each reporting date during the quarterly review process.

**UNIQUE B.V.****Notes to Unaudited Condensed Consolidated Financial Statements****4. Critical Accounting Estimates and Judgments**

The preparation of financial statements in conformity with IFRS requires the Company to make estimates and assumptions that affect the reported amounts and classifications of assets and liabilities, revenues and expenses in the condensed consolidated financial statements. The estimates that have a significant risk of causing a material adjustment to the financial statements are utilized for share-based compensation, income taxes, research and development expenditures and borrowings. Actual results could differ materially from those estimates and assumptions.

The preparation of financial statements in conformity with IFRS also requires the Company to exercise judgment in applying the accounting policies. Critical judgments in the application of the Company's accounting policies relate to research and development expenditures, revenues and the cost of license revenues.

The condensed consolidated financial statements do not include all disclosures for critical accounting estimates and judgments that are required in the annual consolidated financial statements, and should be read in conjunction with the Company's annual consolidated financial statements for the period ended December 31, 2012.

**Revenue recognition**

The Company has not generated any revenues from royalties or product sales through September 30, 2013.

In July 2013, the Company received upfront payments in connection with the Glybera commercialization agreement and hemophilia B co-development agreements. Revenues from such non-refundable, up-front payments are initially reported as deferred revenues on the consolidated balance sheet and are recognized in revenues as earned over the period of the development, commercialization, collaboration or manufacturing obligation.

The Company also generates revenues from collaborative research and development arrangements. Such agreements may consist of multiple elements and provide for varying consideration terms, such as up-front, milestone and similar payments, which require significant analysis by management in order to determine the appropriate method of revenue recognition.

Where such arrangements can be divided into separate units of accounting (each unit constituting a separate earnings process), the arrangement consideration is allocated to the different units based on their relative fair values and recognized over the respective performance period. Where the arrangement cannot be divided into separate units, the individual deliverables are combined as a single unit of accounting and the total arrangement consideration is recognized over the estimated collaboration period. Such analysis requires considerable estimates and judgments to be made by us, including the relative fair values of the various elements included in such agreements and the estimated length of the respective performance periods.

Management has concluded that the up-front payments constitute a single unit of accounting, and accordingly, the up-front payments will be recognized over the estimated remaining period of the related manufacturing technologies.

**5. Seasonality of Operations**

The Company's financial results have varied substantially, and are expected to continue to vary, from quarter to quarter. The Company therefore believes that period-to-period comparisons should not be relied upon as indicative of future financial results. The Company believes that its ordinary activities are not linked to any particular seasonal factors.

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## Notes to Unaudited Condensed Consolidated Financial Statements

### 6. Segment Information

Operating segments are identified on the basis of whether the allocation of resources and/or the assessment of performance of a particular component of uniQure's activities are regularly reviewed by uniQure's chief operating decision maker as a separate operating segment. By these criteria, the activities of uniQure are considered to be one segment, which comprises the discovery, development and commercialization of innovative gene therapies, and the segmental analysis is the same as the analysis for uniQure as a whole. The Management Board is the chief operating decision maker, and it reviews the consolidated operating results regularly to make decisions about the Company's resources, and to assess overall performance.

The Company currently, and in the near future, is expected to derive the substantial majority of its revenues from a single party, Chiesi, based in Italy. The Company and Chiesi have entered into an exclusive collaboration for the development and commercialization of the Company's Glybera and Hemophilia B programs in Europe and certain additional territories, pursuant to agreements which were entered into in April 2013, and which became effective in June 2013.

### 7. Property, Plant and Equipment

	LEASEHOLD IMPROVEMENTS	CONSTRUCTION IN PROCESS	LAB EQUIPMENT	OFFICE EQUIPMENT	TOTAL
	(€ in thousands)				
<b>Period ended September 30, 2013</b>					
Opening net book amount	598	—	270	317	1,185
Additions	—	85	55	426	566
Depreciation charge	(148)	—	(94)	(156)	(398)
<b>Closing net book amount</b>	<b>450</b>	<b>85</b>	<b>231</b>	<b>587</b>	<b>1,353</b>
<b>At September 30, 2013</b>					
Cost	1,264	85	3,014	1,305	5,668
Accumulated depreciation	(814)	—	(2,783)	(718)	(4,315)
<b>Net book amount</b>	<b>450</b>	<b>85</b>	<b>231</b>	<b>587</b>	<b>1,353</b>

Construction in Process ("CIP") at September 30, 2013 relates to the build-out of the manufacturing facility in Lexington, Massachusetts.

Depreciation expense of €398,000 for the nine months ended September 30, 2013 (nine months ended September 30, 2012: €403,000) has been charged in research and development expense.

# UNIQUE B.V.

## Notes to Unaudited Condensed Consolidated Financial Statements

### 8. Intangible Assets

	INTANGIBLE ASSETS (€ in thousands)
<b>Period ended September 30, 2013</b>	
Opening net book amount	3,278
Additions	3,647
Reductions	(155)
Amortization charge	—
<b>Closing net book amount</b>	<b>6,770</b>
<b>At September 30, 2013</b>	
Cost	6,770
Accumulated amortization and impairment	—
<b>Net book amount</b>	<b>6,770</b>

Additions to intangible assets for the nine months ended September 30, 2013 include the capitalization of Glybera development expenses, in accordance with IAS 38, for a total amount of €2,103,000 compared with €nil for the nine months ended September 30, 2012. Capitalization of Glybera costs commenced on March 21, 2013. Other additions relate to the capitalization of sublicense amendment fees following the entry into Chiesi collaboration agreements, for a total amount of €1,544,000 compared with €nil for the nine months ended September 30, 2012. On July 1, 2013, the Company altered the terms of the previous Glybera-related license agreement, entered into in 2012, with Ampliphi Biosciences Corporation, reducing the capitalized amount by €155,000 (CAN\$200,000).

### 9. Other Non-Current Assets

For the nine months ended September 30, 2013, the amount represents a refundable deposit for the Lexington, Massachusetts facility, paid in September 2013.

### 10. Trade and Other Receivables

	DECEMBER 31, 2012	SEPTEMBER 30, 2013
	(€ in thousands)	
Receivables from related parties	26	726
Trade accounts receivable	397	343
Other receivables	—	1,373
Social security and other taxes	418	335
<b>Trade and other receivables</b>	<b>841</b>	<b>2,777</b>

The fair value of trade and other receivables approximates their carrying value. As of September 30, 2013 and December 31, 2012, all trade and other receivables were assessed as fully recoverable. The carrying

**UNIQUE B.V.****Notes to Unaudited Condensed Consolidated Financial Statements**

amount of the Company's trade receivables are fully denominated in Euros. The receivables from related parties as of September 30, 2013 relate to invoiced amounts to Chiesi based on revenue recognized and expenses reimbursed.

The other classes within trade and other receivables do not contain impaired assets. The maximum exposure to credit risk at the reporting date is the carrying value of each class of receivable mentioned above.

The other receivables primarily relate to prepaid rent, insurance and certain annual licence fees for software and Intellectual Property.

**11. Inventories**

	DECEMBER 31, 2012	SEPTEMBER 30, 2013
	(€ in thousands)	
Raw materials	—	145
Work in Process / Intermediate Products	—	282
<b>Inventories</b>	<b>—</b>	<b>427</b>

Inventories as of September 30, 2013 were €427,000 (2012: €nil). The amount includes the raw materials that are to be capitalized in connection with the manufacturing of Glybera for commercial sale, which is expected to commence in the first half of 2014. Also included in inventories are amounts assigned to work in progress and intermediate products following the initial production batches of Glybera. Only Glybera-related material that could not be used for commercial purposes is expensed.

**12. Cash and Cash Equivalents**

	DECEMBER 31, 2012	SEPTEMBER 30, 2013
	(€ in thousands)	
<b>Cash at bank and on hand</b>	<b>263</b>	<b>31,427</b>

The cash balance as of September 30, 2013 reflects the receipt of €17,000,000 in up-front payments from Chiesi (July 2013), €10,000,000 in convertible debt financing from Collier Capital (June 2013), \$10,000,000 in venture debt financing from Hercules Technology Growth Corporation (March 2013) and the drawdown of the remaining advance relating to the December 2012 convertible loan agreement, amounting to €1,999,000.

**Supplemental information relating to the cash flow statement**

The conversion of the €5,000,000 convertible loan, together with accrued interest of €320,000, amounting to €5,320,000 in aggregate represented a non-cash item as of September 30, 2012. The conversion of the €13,497,000 convertible loan, comprising an amount of €1,498,000 drawn down in December 2012 and the balance of €11,999,000 drawn down during 2013, represented a non-cash item as of September 30, 2013. Refer to Note 13 below.

**UNIQUE B.V.****Notes to Unaudited Condensed Consolidated Financial Statements**

The derivative result arising on early conversion of the loan, amounting to €1,333,000 and the derivative result relating to embedded derivatives, amounting to €2,339,000, represented non-cash items as of September 30, 2013.

Purchases of fixed assets and changes in trade and other payables contain a non-cash item of €30,000 largely related to the purchase of fixed assets, which have not yet been paid as of September 30, 2013. Refer to Note 7 above.

**13. Equity**

uniQure was incorporated on January 10, 2012. The comparative period ending September 30, 2012 represents the first accounting period for the Company. On April 5, 2012 uniQure acquired the AMT Business. The business combination of uniQure and the AMT Group is accounted for as a reverse acquisition, and the financial statements of the AMT Business are presented as the financial statements of uniQure, with an adjustment required to reflect the capital of uniQure in accordance with the requirements of IFRS3 in relation to reverse acquisitions (further details are set out in Note 1 of the audited consolidated financial statements of uniQure for the year ended December 31, 2012). The amount recognized as issued equity interests in the consolidated financial statements is determined by the issued equity interest in AMT outstanding immediately prior to the business combination, but the equity structure (the number and type of equity interests issued) reflects the equity structure of uniQure. Accordingly, the share capital and share premium accounts of AMT disclosed in its audited financial statements for prior years are restated as if uniQure ordinary shares had been issued. The exchange ratio of uniQure shares issued for AMT shares was 1-for-1, but because AMT shares had a nominal value of €0.04 and uniQure shares have a nominal value of €0.01, the impact of this approach is to reduce the balance of the share capital reported within the previous AMT accounts and correspondingly increase the balance on the share premium account. Further details are set out in the uniQure financial statements for the year ended December 31, 2012.

On April 5, 2012, uniQure entered into the financing arrangements described above, consisting of the subscription for €6,000,000 in new equity and the conversion of loans plus interest amounting to €5,320,000.

On April 19, 2012, uniQure raised a further €1.0 million through the issue of class A ordinary shares at a price of €0.614 per share to Cooperatieve Gilde Healthcare II U.A., an existing shareholder of uniQure.

During the period covered by these interim financial statements, uniQure had a single class of shares, which are denominated as ordinary shares. Within this class of ordinary shares, there are further sub-denominations between class A Ordinary Shares, class B Ordinary Shares and class C Ordinary Shares. Other than the fact that certain corporate resolutions require the approval of the general meeting of the class A ordinary shares, all classes of shares carry equal economic rights and rank equally.

Following a general meeting of shareholders of uniQure on July 22, 2013, the Company's authorized share capital was increased from €1,900,000 or 190,000,000 shares to €2,000,000 or 200,000,000 shares by the creation of a new sub-denomination of class C Ordinary Shares, on the following basis:

	<b>A</b>	<b>B</b>	<b>C</b>	<b>TOTAL</b>
Number of Ordinary Shares	171,406,311	18,593,689	10,000,000	200,000,000
Value (€)	1,714,063	185,937	100,000	2,000,000

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## Notes to Unaudited Condensed Consolidated Financial Statements

As of September 30, 2013, a total of 60,948,978 shares were issued and paid up in full at a nominal value of €0.01 per share (December 31, 2012: 48,267,493 shares at €0.01 per share).

The shares issued during the nine months ended September 30, 2013, the numbers of shares issued, and the impact on the share capital and the share premium is as follows:

<u>Date</u>	<u>Description</u>	<u>Sub-class of ordinary shares</u>	<u>Number of shares</u>	<u>Share capital Amounts</u>	<u>Share premium Amounts</u>	<u>Total equity Amounts</u>
(€ in thousands)						
January 1, 2012	Brought forward		23,748,127	237	99,947	100,184
January 4, 2012	Investment in AMT ordinary shares		7,352,938	74	2,426	2,500
April 5, 2012	Forbion conversion of existing convertible loan plus interest	A	5,320,000	53	5,267	5,320
April 5, 2012	Forbion new equity investment	A	9,771,987	98	5,902	6,000
April 18, 2012	Gilde new equity investment	A	1,628,664	16	984	1,000
<b>September 30, 2012</b>			<b>47,821,716</b>	<b>478</b>	<b>114,526</b>	<b>115,004</b>
November–December, 2012	Employees and other persons new equity investment	B	445,777	5	269	274
<b>December 31, 2012</b>			<b>48,267,493</b>	<b>483</b>	<b>114,795</b>	<b>115,278</b>
January–May, 2013	Employees and other persons new equity investment	B	453,737	4	274	278
July 24, 2013	Chiesi new equity investment	C	5,546,070	55	13,945	14,000
July 26, 2013	Conversion of 2012 & 2013 convertible loans	A	6,681,678	67	13,430	13,497
<b>September 30, 2013</b>			<b>60,948,978</b>	<b>609</b>	<b>142,444</b>	<b>143,053</b>

## UNIQUE B.V.

### Notes to Unaudited Condensed Consolidated Financial Statements

Analysis of the shares issued between cash and non-cash items for the nine months ended September 30, 2013:

	Description	Sub-class of ordinary shares	Cash items	Non cash items	Total
(€ in thousands)					
<b>2012</b>					
January 4, 2012	Investment in AMT ordinary shares		2,500	—	2,500
April 5, 2012	Forbion new equity investment	A	6,000	—	6,000
April 5, 2012	Forbion conversion of existing convertible loan plus interest	A	—	5,320	5,320
April 19, 2012	Gilde new equity investment	A	1,000	—	1,000
<b>September 30, 2012</b>			<b>9,500</b>	<b>5,320</b>	<b>14,820</b>
November-December, 2012	Employees and other persons new equity investment	B	274	—	274
<b>December 31, 2012</b>			<b>9,774</b>	<b>5,320</b>	<b>15,094</b>
<b>2013</b>					
January-May, 2013	Employees and other persons new equity investment	B	278		278
July 24, 2013	Chiesi new equity investment	C	14,000		14,000
July 26, 2013	Conversion of 2012 & 2013 convertible loans	A		13,497	13,497
<b>September 30, 2013</b>			<b>14,278</b>	<b>13,497</b>	<b>27,775</b>

Further details of the shares issued in 2012 are described in the 2012 audited financial statements. For further details about the conversion of the convertible loan in July 2013 refer to Note 14.

During the nine months ended September 30, 2013 and during the year ended December 31, 2012, no new shares were issued upon the exercise of share options. On December 31, 2012 and September 30, 2013 36,294 shares were held by the stichting participatie AMT as treasury shares. The par value as of September 30, 2013 was €0.01 per share (as of December 31, 2012: €0.01 per share). All shares issued by the Company were fully paid. Besides the minimum amount of share capital to be held under Dutch law, there are no distribution restrictions applicable to equity of the Company.

#### Share Premium

During the year ended December 31, 2012, the Company entered into a reverse acquisition that is described in the annual consolidated audited financial statements for the year ended December 31, 2012.

Total additions to share premium during the nine months ended September 30, 2013 were €27,649,000 net of costs. This increase in share premium was due to the issue of shares as described above.

#### Other Reserves

Accumulated expense related to the AMT share option plan for the period up to April 5, 2012, amounting to €2,987,000, is offset against the retained losses at April 5, 2012 following the termination of the AMT share option scheme, as set out in the Consolidated Statement of Changes in Equity and as described further in the consolidated audited financial statements for the year ended December 31, 2012.



**UNIQUE B.V.****Notes to Unaudited Condensed Consolidated Financial Statements**

The costs of equity-settled share-based payments to employees are recognized in the income statement, together with a corresponding increase in equity during the vesting period, taking into account (deferral of) corporate income taxes.

During the nine months ended September 30, 2013 the Company recognized a share-based payment expense of €1,411,000 (nine months ended September 30, 2012: €1,228,000), as described in Note 20 below and the accumulated expense of the share incentive plan recognized in the income statement is shown separately in the equity category Other Reserves in the Consolidated Statement of Changes in Equity.

As per Note 15 below, the Company also presented in other reserves the result of the conversion of the convertible loan to the amount of €3,005,000

In the period presented in these unaudited consolidated financial statements, the Company did not have any legal or other types of restricted reserves.

**14. Trade and Other Payables**


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	DECEMBER 31, 2012	SEPTEMBER 30, 2013
	(€ in thousands)	
Trade payables	2,099	3,198
Social security and other tax	152	763
Other current liabilities	1,816	2,613
<b>Total trade and other payables</b>	<u>4,067</u>	<u>6,574</u>

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***Other current liabilities***

As of September 30, 2013 and December 31, 2012, other current liabilities consisted principally of accruals for services provided by vendors but not yet billed, reimbursements received from research and development partners for expenses which have yet to be incurred and miscellaneous liabilities.

# UNIQUE B.V.

## Notes to Unaudited Condensed Consolidated Financial Statements

### 15. Borrowings

	DECEMBER 31, 2012	SEPTEMBER 30, 2013
	(€ in thousands)	
<b>Non-current</b>		
Borrowings	—	7,291
Finance lease liabilities	450	342
<b>Total non-current</b>	<b>450</b>	<b>7,633</b>
<b>Current</b>		
Debt to related party—Financial liability	1,366	—
Debt to related party—Embedded derivative	132	892
Borrowings—Embedded derivative	—	273
<b>Total current</b>	<b>1,498</b>	<b>1,165</b>
<b>Total</b>	<b>1,948</b>	<b>8,798</b>

#### December 2012 Convertible Loan and Amendment in March 2013

On December 17, 2012, uniQure entered into a convertible loan agreement with four of its major shareholders (Forbion, Gilde, Grupo Netco and Lupus Alpha), in respect of unsecured and unsubordinated loan notes, which have an issue price of 100% and pay an annual coupon of 8%. Of the total loan €1,498,000 was drawn down in the period to December 31, 2012 and the balance of €1,999,000 was drawn down in the period from January 1, 2013 to January 31, 2013, amounting to a total convertible loan amount of €3,497,000.

In March 2013, uniQure increased the loan by an additional €10,000,000 investment by Collier Capital. As part of the increase, the loan note terms for all loan note holders described in the annual consolidated financial statements were amended such that the final maturity date of the loan notes was extended to December 31, 2014. Additionally, the warrant entitlement was reduced to 10% of the principal amount of the loan provided to uniQure.

Following the subscription for new equity by Chiesi, on July 21, 2013 the full convertible loan was converted on July 26, 2013 into new Class A Ordinary Shares, at a conversion price of €2.02 per share. This conversion marked the extinction of the convertible derivative instrument. The remaining derivative element arises from the warrants issued to the holders of the convertible loan as part of the convertible loan arrangements.

The warrants associated with the convertible loan, and which survive the conversion of the loan, are presented in the consolidated Balance Sheet as at September 30, 2013 within liabilities as an embedded derivative with a fair value of €892,000.

The elimination of the embedded derivative (convertible element) by the early conversion of the loan created €3,005,000 of Other Reserves within the Equity presentation.

During the nine months ended September 30, 2013, an amount of €4,676,000 (compared with €545,000 for the nine months ended September 30, 2012) was recorded as finance expense. This amount relates to €3,716,000 of derivative results (compared with €526,000 for the nine months ended September 30,

**UNIQUE B.V.****Notes to Unaudited Condensed Consolidated Financial Statements**

2012) and the remainder consists of interest expense in relation to the convertible note, Hercules borrowing and interest expense on the financial lease.

**Hercules Borrowing**

The presented non-current borrowings relate to the Hercules Technology Growth Corp. venture debt loan facility, entered into on June 14, 2013 for a book value of €7,291,000 as of September 30, 2013, presented net of expenses for facility charges of 1.25% plus expenses related to legal counsel. The loan commitment is \$10 million with an interest rate of 11.85% and a back-end fee of 3.45%, which matures over a period of 39 months from the loan closing date. The interest-only period was initially set at 9 months and was extended to 15 months on completion of the transaction with Chiesi. In addition, the loan is secured by a lien on all of the Company's assets (excluding intellectual property).

The warrant included in this loan agreement is not closely related to the host contract and therefore has been split and accounted for separately as a financial derivative measured at fair value through profit or loss. The fair value of this embedded derivative is €273,000 and is included within the Current liabilities: Borrowings—embedded derivative on the Consolidated Balance Sheet as of September 30, 2013.

**Finance Lease Liability**

The finance lease liability relates to the Company's facility at the Meibergdreef in Amsterdam, the Netherlands.

The condensed consolidated financial statements do not include all disclosures for borrowings that are required in the annual consolidated financial statements, and should be read in conjunction with the Company's annual consolidated financial statements for the period ended December 31, 2012.

**16. Revenues and Deferred Revenues**

	<b>DECEMBER 31, 2012</b>	<b>SEPTEMBER 30, 2013</b>
	<b>(€ in thousands)</b>	
License Revenues	—	220
Collaboration Revenues	—	1,831
Deferred License Revenues Current Portion	—	1,079
Deferred License Revenues	—	15,899

During the nine months ended September 30, 2013, an amount of €220,000 (nine months ended September 30, 2012: €nil) was recognized as license revenues. This amount relates to the recognition of the up-front payments received from Chiesi. During the nine months ended September 30, 2013, an amount of €1,831,000 (nine months ended September 30, 2012: €nil) was recognized as collaboration revenues. This amount related to certain approved activities the Company was able to recharge and reimbursements of expenses under its Co-Development Agreement with Chiesi in respect of its Hemophilia B program.

Upon signing of the Commercialization Agreement and the Co-Development and Commercialization Agreement with Chiesi on April 29, 2013, the Company received €17,000,000 as a non-refundable upfront payment. Based on an assessment performed to the Company, the €17,000,000 will be amortized on a straight-line basis, and presented as license revenues, over a period from July 2013 through September

**UNIQUE B.V.****Notes to Unaudited Condensed Consolidated Financial Statements**

2032: the date of expiration of the last intellectual property protection related to the manufacturing process. The Company determined that the €17,000,000 of up-front payments received from Chiesi constituted a single unit of accounting. The up-front payments related to licenses and reimbursement of past development costs for Glybera and hemophilia B as follows:

- 1) €2,000,000—Reimbursement of past development costs related to Glybera. Continuing performance obligation: maintaining the market authorization for Glybera (including the post-approval commitment to conduct the Phase IV study);
- 2) €5,000,000—for past development costs related to hemophilia B. Continuing performance obligation: complete the Co-Development program and file for Marketing Authorization in the European Union;
- 3) €10,000,000—for having set up an EMA approved manufacturing/production facility. Continuing performance obligation: supply of commercial product to Chiesi.

Although the Company believes that the different elements have different cost levels, the Company is not able to properly estimate the respective fair values of the various elements. Therefore, the Company has concluded that the three deliverables within the arrangement are linked in such a way that the commercial effect cannot be understood without reference to the series of transactions as a whole. Therefore, the individual performance obligations were combined as a single unit of accounting and the total arrangement consideration will be recognized over the estimated life of the agreements under which the continuing performance obligations exist.

The elements described above are based on the current assumption that hemophilia B is anticipated to receive regulatory approval in late 2018, and that the commercial launch is within 3 months following approval. Based on the above, best estimate of the anticipated duration of the agreements is in line with the expiration term of the patent for manufacturing of commercial product which is 19 years. Based on the aforementioned facts, the Company has deferred the revenue and will recognize the €17,000,000 of up-front payments as license revenue on a straight-line basis over 19 years.

For the nine months ended September 30, 2013, the Company recognized an expense, under Costs of goods sold, in relation to its obligation to repay to the Dutch Government a portion of a grant received between 2001 and 2005 in connection with the development of Glybera; the amount was calculated as an agreed 40% of the upfront payment received in relation to Glybera. See a further description under Note 23, Contingent Liabilities.

Collaboration revenues from contracts, typically from delivering research and development services, relate to the agreements, and is recognized on the basis of labor hours delivered at the Agreements' full time employee rate.

Cost reimbursements to which the Company is entitled to under agreements are also recognized as collaboration revenues in the income statement in the same quarter of the recorded cost they intend to compensate, except for reimbursement of certain expenses incurred in the periods prior to the completion of the Chiesi agreements (on June 30, 2013); such revenues are recognized at the moment that Chiesi incurred the obligation to reimburse them, i.e. on June 30, 2013. When the reimbursable costs are not yet invoiced these amounts are included as a component of trade and other receivables on the balance sheet.

**UNIQURE B.V.****Notes to Unaudited Condensed Consolidated Financial Statements****17. Research and development expenses**

Research and development expenses mainly increased due to the additional development and clinical activities required to support the planned commercial launch of Glybera, as well as the progression of uniQure's other programs through late stage research and clinical development.

**18. General and administrative expenses**

General and administrative expenses increased to €7,612,000 for the nine months ended September 30, 2013 from €4,438,000 for the nine months ended September 30, 2012. The increase is primarily due to expenses related to consultants (commercial, operations and administrative) and professional fees.

**19. Other Comprehensive Income**

For the nine months ended September 30, 2013 the amount shown as €6,000 represents the foreign currency translation arising from the US subsidiary, which was established in 2013 (for the nine months ended September 30, 2012: €nil).

**20. Share-Based Payments**

The condensed consolidated financial statements do not include all disclosures for share-based payments that are required in the annual consolidated financial statements, and should be read in conjunction with the Company's annual consolidated financial statements for the period ended December 31, 2012.

During the nine months ended September 30, 2013 the Company recognized a share-based payment expense of €1,411,000 (nine months ended September 30, 2012: €1,228,000). The share-based payment expense in the first quarter of 2012 was based on the AMT plan only. During the second quarter of 2012, the uniQure share-based payment plan was introduced, which resulted in a higher expense charge than the previous quarter. For the nine months ended September 30, 2013, employee payroll headcount increased from 50 to 79, which lead to the additional increase in share-based payment expense during the period.

**21. Loss Per Share****Basic**

Basic loss per share is calculated by dividing the loss attributable to equity holders of the Company by the weighted average number of issued and outstanding ordinary and preferred shares during the year.

	<b>NINE MONTHS ENDED SEPTEMBER 30,</b>	
	<b>2012</b>	<b>2013</b>
	<b>(€ in thousands)</b>	
Loss attributable to equity holders of the Company	(10,424)	(20,422)
Weighted average number of ordinary shares outstanding	42,155,570	52,971,836

**UNIQUE B.V.****Notes to Unaudited Condensed Consolidated Financial Statements*****Diluted***

Diluted loss per share is calculated by adjusting the weighted average number of ordinary shares outstanding to assume conversion of all dilutive potential ordinary shares. Due to the fact that the Company is loss making, all potential ordinary shares had an antidilutive effect, if converted, and thus have been excluded from the computation of loss per share.

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	<u>DECEMBER 31, 2012</u>	<u>SEPTEMBER 30, 2013</u>
	(€ in thousands)	
Share options		
<b>Total</b>	<u>8,031,777</u>	<u>8,451,110</u>

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**22. Related-Party Transactions**

In the nine month periods ended September 30, 2013 and 2012, the Management Board received regular salaries and contributions to post-employment schemes. Additionally, selected members of the Supervisory Board received compensation for their services in the form of cash compensation.

Funds affiliated with Forbion Capital partners have a material interest in the Company. In addition, Professor Sander van Deventer and Mr. Sander Slootweg, who were appointed as members of the Supervisory Board of uniQure on April 5, 2012, are each partners of Forbion. Based on the information above, Forbion is a related party of uniQure.

Funds affiliated with Gilde Healthcare have a material interest in the Company. In addition, Mr. Edwin de Graaf, who was appointed as a member of the Supervisory Board of uniQure on April 5, 2012, is a partner of Gilde Healthcare Partners. Based on the information above, Gilde Healthcare is a related party of uniQure.

Funds affiliated with Grupo Netco and Lupus Alpha also have material interests in the company. Chiesi became a related party following the the commercial and investment agreements concluded with the Company on June 30, 2013, and Collier Capital became a related party following the conversion of the convertible loan in July 2013.

**Transactions**

The related parties identified above participated in the following transactions during the nine month periods ended September 30, 2013 and September 30, 2012.

The 2009 convertible loan from Forbion accrued interest of 5% (a finance charge of €70,000), during the period from January 1, 2012 until its conversion on April 5, 2012. No interest in respect of this loan arose in respect of the nine months ended September 30, 2012 because the loan had already been previously converted into 5,320,000 class A ordinary shares.

The 2012 convertible loan from Forbion, Gilde, Lupus Alpha, Grupo Netco and affiliates, and Collier Capital, as amended in March 2013, generated in the nine months ending September 30, 2013 generated a combined funding of €11,998,000. This loan accrued interest of 8% up until the date of conversion in July 2013 (plus an amount up to the interest payment date), amounting to a total interest amount payable of €434,000.

## UNIQUE B.V.

### Notes to Unaudited Condensed Consolidated Financial Statements

In the period ending September 30, 2013, the Company received various payments from Chiesi comprising a subscription for ordinary C shares of €14,000,000 and up-front commercial payments of €16,875,000. In addition, the Company received funds from Chiesi for issued invoices totalling €725,000.

As of September 30, 2013 the Company had a receivable outstanding with Chiesi for €726,000.

#### Key Management Compensation

The below table shows the compensation for the Supervisory Board, the Managing Directors and senior Management:

FOR THE		SHORT TERM EMPLOYEE BENEFITS	SHARE- BASED PAYMENTS	POST- EMPLOYMENT BENEFITS (€ in thousands)	ADVISORS FEES	TERMINATION BENEFITS	TOTAL
Year ended December 31, 2012	Supervisory Board	—	134	—	121	—	255
	Managing directors	695	509	92	—	—	1,296
	Senior Management	689	452	41	—	—	1,182
		<b>1,384</b>	<b>1,095</b>	<b>133</b>	<b>121</b>	—	<b>2,733</b>
9 months ended September 30, 2012	Supervisory Board	—	79	—	93	—	172
	Managing directors	428	337	69	—	—	834
	Senior Management	488	297	31	—	—	816
		<b>916</b>	<b>713</b>	<b>100</b>	<b>93</b>	—	<b>1,822</b>
9 months ended September 30, 2013	Supervisory Board	—	211 <sup>(2)</sup>	—	49	—	260
	Managing directors	577 <sup>(1)</sup>	325	45	—	—	947
	Senior Management	753	335	78	—	—	1,166
		<b>1,330</b>	<b>871</b>	<b>123</b>	<b>49</b>	—	<b>2,373</b>

<sup>(1)</sup> The Management board received Management bonuses

<sup>(2)</sup> For one Supervisory Board member, the Options were granted late in 2012, but assumed an April 2012 grant date for purposes of vesting

The condensed consolidated financial statements do not include all disclosures for related-party transactions that are required in the annual consolidated financial statements, and should be read in conjunction with the Company's annual consolidated financial statements for the period ended December 31, 2012.

#### 23. Commitments / Contingent Liabilities

uniQure leases various office space and laboratory space under operating lease agreements. The Company leases its headquarters under an agreement between uniQure and AMC, represented by BDDA and Amsterdam Vector Productions B.V. ("AVP"), both subsidiaries of AMC (Second Rental Agreement) in respect of facilities located at Meibergdreef 61 Amsterdam, from October 1, 2005 until September 30, 2016, and an agreement for the lease of facilities at Meibergdreef 57, Amsterdam, from July 1, 2006 until September 30, 2016. The aggregate annual lease payments amount to €542,000.

The lease expenditure charged to the income statement for the nine months ended September 30, 2013 was €471,000 (for the nine months ended September 30, 2012: €526,000).

## UNIQUE B.V.

### Notes to Unaudited Condensed Consolidated Financial Statements

The future aggregate minimum lease payments under non-cancellable operating leases as of September 30, 2013 and December 31, 2012 are as follows:

	DECEMBER 31, 2012	SEPTEMBER 30, 2013
	(€ in thousands)	
No later than 1 year	542	542
Later than 1 year and no later than 5 years	1,627	1,220
<b>Total</b>	<b>2,169</b>	<b>1,762</b>

On July 24, 2013 uniQure entered into an agreement for the lease of facilities at 113 Hartwell Avenue, Lexington, Massachusetts, United States from November 5, 2013 until November 5, 2023. uniQure has an option to extend the lease for up to an additional 10 years. The aggregate annual lease payments for the period to November 5, 2023 amount to \$18,937,000, including an initial rent-free period of seven months from the commencement of the lease. Because the lease period commences after the period covered by these condensed financial statements, there is no financial impact on the period covered by these financial statements. As of September 30, 2013, the Company considered the Lexington lease obligations a contingency and not yet a commitment.

Further details regarding the accounting for this lease, including the costs of arranging the lease (which amounted to \$52,000 and which will be recognized over the duration of the lease) and certain improvements undertaken by the landlord (which will amount to \$7,207,000 and which will be accounted for as an incentive to enter into the lease and accordingly taken as a benefit to the profit and loss account over the duration of the lease) will be set out in the audited consolidated financial statements for the year ending December 31, 2013.

### Research and Development Commitments

uniQure has entered into research and development commitments in relation to uniQure's product pipeline. The future aggregate minimum payments under these research and development commitments are as follows:

	DECEMBER, 2012	SEPTEMBER 30, 2013
	(€ in thousands)	
No later than 1 year	277	298
Later than 1 year and no later than 5 years	—	—
Later than 5 years	—	—
<b>Total</b>	<b>277</b>	<b>298</b>



**UNIQUE B.V.****Notes to Unaudited Condensed Consolidated Financial Statements****Grant Commitments**

From October 1, 2000 until May 31, 2005, AMT received a technical development loan from the Dutch government in relation to development of Glybera. This grant includes a repayment clause in the event the Company generates revenues from the related project. AMT received total grants of €3,605,000 relating to eligible project costs in the grant period. The grant amount received bears interest of 5.7% per annum and must be repaid in the period January 1, 2008 through December 31, 2017 as a percentage of revenues which are derived from product sales of Glybera. If future royalty payments are not sufficient to repay the grant on or prior to December 31, 2017, or if there are no revenues generated, the remaining balance will be forgiven. Repayment obligations continue to apply if the product is not commercialized or transferred to others. The total amount of the contingent commitment as at September 30, 2013 was €5,433,000 comprising the original total amount of the grant together with accrued interest. During the nine months ended September 30, 2013 the Company recognized an amount of €800,000 as a charge in the consolidated statement of comprehensive income within Costs of goods sold. This amount was paid to the Dutch Government in September 2013 and was calculated as 40% of the upfront amount received specifically related to Glybera.

Historically, the Company also received a "Technisch ontwikkelingsproject" (TOP) (or technical development project) grant from the Dutch government amounting to €130,000 on a project that was terminated. If the Company realizes income from the sale of assets developed under that grant, repayment clauses will apply. The Company has not recorded any liability to repay amounts in respect of this grant within these financial statements.

On January 5, 2010, the Company was awarded an investment credit (innovatiekrediet) from the Dutch government (Ministry of Economic Affairs—Agentschap.nl) in respect of the Company's program for Duchenne Muscular Dystrophy. The credit covers 35% of the costs incurred in respect of the program up to a maximum of €4,000,000. The credit includes a repayment clause dependent on the technical success of this program (which is expected to be demonstrated if the product can be successfully commercialized). The credit is interest-bearing at a rate of 11.4% per annum. To date, the Company has received €729,000 under this investment credit, and as of December 31, 2012, the total amount of the liability was €956,000, representing the amount of the original advance together with accrued interest (2011: €858,000). The credit was to be repaid after the funded part of the program was completed in 2013, out of a percentage of revenues derived from the sales resulting from the Company's Duchenne Muscular Dystrophy program. The assets which are financed by means of the investment credit are subject to a right of pledge for the benefit of the Dutch Ministry of Economic Affairs. The project has been terminated following failure to achieve the scientific goals and the Company does not anticipate that any amounts will be realized from this project and consequently there will not be any obligation to repay any of these amounts.

**Other contingent liabilities**

On October 22, 2013, the Company received a demand letter from Extera Partners, a consulting firm based in Boston, Massachusetts, regarding certain fees alleged to be owed by the Company in respect of consulting services provided in connection with the Company's collaboration agreements with Chiesi, under an engagement which expired on December 31, 2012. The total amount claimed by Extera Partners for present and future fees allegedly due as a result of the Company's collaboration agreements with Chiesi, which were entered into in the second quarter of 2013, is said to be in the order to €7,000,000 to €8,000,000; the engagement letter with Extera contained a cap limiting the maximum payment to €5,000,000. On December 11, 2013, we received a notification of formal request for arbitration by Extera. The Company intends to defend the claim vigorously. The Company has reviewed the demand with counsel

**UNIQUE B.V.**

**Notes to Unaudited Condensed Consolidated Financial Statements**

and believes that the claim is without merit, and consequently it is not expected to have financial consequences for the Company.

**24. Events After the Balance Sheet Date**

On October 22, 2013, the Company received a demand letter from Extera Partners, a consulting firm based in Boston, Massachusetts, as described in Note 23 above. This is not expected to have financial consequences for the Company. No other events occurred after the balance sheet date that would have a material impact on the results or financial position of uniQure.

In October 2013, the Company entered into additional preconstruction commitments to complete the design and planning work, to acquire the building permit and to order certain materials and equipment that require a longer lead time. The combined sum of these additional commitments entered into amounted to \$4,870,000 that is expected to be paid partially in the fourth quarter of 2013 and partially in early 2014.

## **Report of Independent Registered Public Accounting Firm**

To the Board of Directors and shareholders of uniQure B.V.:

In our opinion, the accompanying consolidated balance sheets and the related consolidated balance sheets and the related consolidated statements of comprehensive income, of changes in equity and of cash flows present fairly, in all material respects, the financial position of uniQure B.V. and its subsidiaries at December 31, 2012, December 31, 2011, and December 31, 2010, and the results of their operations and their cash flows for each of the two years in the period ended December 31, 2012 in conformity with International Financial Reporting Standards as issued by the International Accounting Standards Board and in conformity with International Financial Reporting Standards as adopted by the European Union. These financial statements are the responsibility of the Company's management. Our responsibility is to express an opinion on these financial statements based on our audits. We conducted our audits of these statements in accordance with the standards of the Public Company Accounting Oversight Board (United States) and International Standards on Auditing. Those standards require that we plan and perform the audit to obtain reasonable assurance about whether the financial statements are free of material misstatement. An audit includes examining, on a test basis, evidence supporting the amounts and disclosures in the financial statements, assessing the accounting principles used and significant estimates made by management, and evaluating the overall financial statement presentation. We believe that our audits provide a reasonable basis for our opinion.

/s/PricewaterhouseCoopers Accountants N.V.

PricewaterhouseCoopers Accountants N.V.  
Utrecht, The Netherlands  
October 25, 2013

**UNIQUE B.V.**
**Consolidated Balance Sheets**  
 (€ in thousands)

		AS AT DECEMBER 31,		
	NOTES	2010	2011	2012
<b>Assets</b>				
<b>Non-current assets</b>				
Intangible assets	(6)	2,916	2,725	3,278
Property, plant and equipment	(7)	1,286	895	1,185
<b>Total Non-current assets</b>		<b>4,202</b>	<b>3,620</b>	<b>4,463</b>
<b>Current assets</b>				
Receivables from related parties	(8, 24)	35	35	26
Social security and other taxes	(8)	409	249	418
Other receivables	(8)	198	800	397
Cash and cash equivalents	(9)	17,859	1,100	263
<b>Total Current assets</b>		<b>18,501</b>	<b>2,184</b>	<b>1,104</b>
<b>Total assets</b>		<b>22,703</b>	<b>5,804</b>	<b>5,567</b>
<b>Equity</b>				
Share capital		235	237	483
Share premium		99,841	99,947	114,795
Other reserves		1,788	2,728	1,508
Accumulated deficit		(88,205)	(105,505)	(117,234)
<b>Total equity</b>	(10)	<b>13,659</b>	<b>(2,593)</b>	<b>(448)</b>
<b>Liabilities</b>				
<b>Non-current liabilities</b>				
Financial lease liabilities	(11)	221	180	450
Debt to related party	(12, 24)	4,621	4,544	—
<b>Non-current liabilities</b>		<b>4,842</b>	<b>4,724</b>	<b>450</b>
<b>Current liabilities</b>				
Trade payables	(13)	1,556	1,736	2,099
Social security and other taxes	(13)	196	713	152
Debt to related party—financial liability	(12)	—	—	1,366
Debt to related party—embedded derivative	(12)	—	—	132
Other current liabilities	(13)	2,450	1,224	1,816
<b>Total Current liabilities</b>		<b>4,202</b>	<b>3,673</b>	<b>5,565</b>
<b>Total liabilities</b>		<b>9,044</b>	<b>8,397</b>	<b>6,015</b>
<b>Total equity and liabilities</b>		<b>22,703</b>	<b>5,804</b>	<b>5,567</b>

The accompanying notes form an integral part of the consolidated financial statements.

UNIQUE B.V.

**Consolidated Statements of Comprehensive Income**  
(€ in thousands, except share data and per share data)

	NOTE	YEAR ENDED DECEMBER 31,	
		2011	2012
		€—	€—
<b>Revenues:</b>			
License revenues		—	—
Collaboration revenues		—	—
<b>Total revenues</b>		—	—
Cost of goods sold		—	—
<b>Gross loss</b>		—	—
Other income	(14)	2,192	649
Research and development expenses		(15,500)	(10,231)
General and administrative expenses		(3,807)	(4,564)
Other losses—net		(26)	(45)
<b>Total operating costs</b>	(15)	<b>(19,333)</b>	<b>(14,840)</b>
<b>Operating result</b>		<b>(17,141)</b>	<b>(14,191)</b>
Finance income	(17)	277	22
Finance expense	(17)	(436)	(547)
		<b>(159)</b>	<b>(525)</b>
<b>Result before corporate income taxes</b>		<b>(17,300)</b>	<b>(14,716)</b>
Corporate income taxes	(18)	—	—
<b>Net loss (Attributable to equity holders of the Company)</b>		<b>(17,300)</b>	<b>(14,716)</b>
		<b>(17,300)</b>	<b>(14,716)</b>
Other comprehensive income		—	—
<b>Total comprehensive loss*</b>		<b>(17,300)</b>	<b>(14,716)</b>
<b>Loss per share attributable to the equity holders of the Company during the year</b>			
<b>Basic and diluted loss per share</b>	(19)	<b>(0.73)</b>	<b>(0.34)</b>

\* Total comprehensive loss is fully attributable to equity holders of the Company.

The accompanying notes form an integral part of the consolidated financial statements.

**UNIQUE B.V.**
**Consolidated Statements of Changes in Equity**  
 (€ in thousands)

	NOTE	ATTRIBUTABLE TO EQUITY HOLDERS OF THE COMPANY				
		SHARE CAPITAL	SHARE PREMIUM RESERVE	OTHER RESERVES	ACCUMULATED DEFICIT	TOTAL EQUITY
<b>Balance at January 1, 2011</b>	(5)	<b>235</b>	<b>99,841</b>	<b>1,788</b>	<b>(88,205)</b>	<b>13,659</b>
Result for the year		—	—	—	(17,300)	(17,300)
Capital contributions	(5)	2	106	—	—	108
Share-based payment expenses		—	—	940	—	940
<b>Balance at December 31, 2011</b>	(5)	<b>237</b>	<b>99,947</b>	<b>2,728</b>	<b>(105,505)</b>	<b>(2,593)</b>
Balance at January 1, 2012		237	99,947	2,728	(105,505)	(2,593)
Result for the year		—	—	—	(14,716)	(14,716)
Capital contributions	(10)	246	14,848	—	—	15,094
Share-based payment expenses relating to the AMT share option scheme	(10)	—	—	259	—	259
Adjustment to reserves on expiration of the AMT option scheme	(10)	—	—	(2,987)	2,987	—
Share-based payment expenses relating to the uniQure share option scheme	(10)	—	—	1,508	—	1,508
<b>Balance at December 31, 2012</b>		<b>483</b>	<b>114,795</b>	<b>1,508</b>	<b>(117,234)</b>	<b>(448)</b>

The accompanying notes form an integral part of the consolidated financial statements.

UNIQUE B.V.

Consolidated Statements of Cash Flow  
(€ in thousands)

	NOTES	YEAR ENDED DECEMBER 31,	
		2011	2012
<b>Cash flow from operating activities</b>			
Result before corporate income tax		(17,300)	(14,716)
Adjustments for:			
—Depreciation	(15)	590	548
—Impairment of assets	(6)	300	—
—Derivative result	(17)	(207)	(22)
—Derivative result arising on early conversion of the loan	(17)	—	464
—Exchange result	(15)	26	45
—Share-based payment expenses	(16)	940	1,767
—Changes in trade and other receivables	(8)	(442)	243
—Changes in trade and other payables	(13)	(1,039)	180
—Movement on other liabilities	(11)	64	161
—Interest (income)/expense	(17)	365	61
Cash used in operations		(16,703)	(11,269)
Interest paid		(2)	(8)
<b>Net cash used in operating activities</b>		<b>(16,705)</b>	<b>(11,277)</b>
<b>Cash flow from investing activities</b>			
Purchases of property, plant and equipment	(7)	(200)	(392)
Purchases of intangible assets	(6)	(109)	(553)
Interest received	(17)	147	113
<b>Net cash used in investing activities</b>		<b>(162)</b>	<b>(832)</b>
<b>Cash flow from financing activities</b>			
Capital contribution from shareholders	(10, 21)	108	9,774
Convertible loans drawn down	(12)	—	1,498
<b>Net cash generated from financing activities</b>		<b>108</b>	<b>11,272</b>
<b>Net decrease in cash, cash equivalents and other bank overdrafts</b>		<b>(16,759)</b>	<b>(837)</b>
<b>Cash, cash equivalents and bank overdrafts at the beginning of the year</b>	(9)	<b>17,859</b>	<b>1,100</b>
<b>Cash, cash equivalents at the end of the year</b>	(9)	<b>1,100</b>	<b>263</b>

The accompanying notes form an integral part of the consolidated financial statements.

**UNIQUE B.V.****Notes to Consolidated Financial Statements****1. General Information****uniQure B.V.**

uniQure B.V. ("uniQure" or the "Company") is a biopharmaceutical company, incorporated and domiciled in the Netherlands, with its headquarters at Meibergdreef 61, 1105 BA, Amsterdam. The Company is a leader in the field of gene therapy, with the first product to receive regulatory approval in the European Union and with multiple collaborations designed to accelerate the development of a pipeline of additional product candidates. The Company was incorporated in January 2012 to acquire and continue the gene therapy business ("AMT Business") of Amsterdam Molecular Therapeutics (AMT) Holding N.V. ("AMT") and its subsidiaries (collectively, the "AMT Group") and to facilitate additional financing, as described further below. As used in these financial statements, unless context indicates otherwise, all references to "uniQure" or the "Company" refer to uniQure and its consolidated subsidiaries.

**Amsterdam Molecular Therapeutics (AMT) Holding N.V., prior to April 5, 2012**

AMT was, until its liquidation (as described further below in this Note 1), incorporated and domiciled in the Netherlands. It was founded by scientists who were investigating gene therapy approaches for the treatment of lipoprotein lipase deficiency ("LPLD") at the Academic Medical Center (the "AMC") of the University of Amsterdam. The work on LPLD led to the development of Glybera® as the AMT Business's lead program. In December 2009, AMT submitted a Marketing Authorization Application ("MAA") for Glybera to the European Medicines Agency ("EMA").

In 2011, following the Committee for Human Medicinal Products ("CHMP") recommendation to the EMA to refuse the MAA for Glybera, AMT was forced to evaluate its strategic options. Under its restructuring plan announced in late 2011, AMT reduced its number of employees from approximately 100 to approximately 45 by early 2012.

Also by late 2011, AMT's financial resources were depleted and AMT faced significant challenges in raising additional funding from new investors. On December 27, 2011, AMT entered into an agreement to issue 7,352,938 new ordinary shares to existing investors at a price of €0.34 per share, raising a total of €2.5 million. These funds provided additional time for AMT's management and Supervisory Board to explore opportunities to fund the AMT Business.

AMT designed a transaction structure to transfer the AMT Business into a new, unlisted company which could then be funded by Forbion Co-Investment II Cooperatief U.A. and Forbion Co-Investment Cooperatief U.A., which we refer to collectively as "Forbion".

**Formation of uniQure and combination with the AMT Business on April 5, 2012**

On February 17, 2012, AMT announced that it had entered into a conditional agreement with the newly created entity, uniQure, under which AMT agreed to transfer its entire interest in the AMT Business. uniQure was a newly formed company that issued equity shares to the existing shareholders of AMT in exchange for the transfer of the AMT Business, such that there was no change in the substance of the reporting entity.

The proposed transaction between uniQure and AMT was approved at a meeting of AMT shareholders on March 30, 2012 and completed on April 5, 2012.



## UNIQUE B.V.

### Notes to Consolidated Financial Statements

uniQure issued 31,101,065 class B ordinary shares with a nominal value of one euro cent ("class B ordinary shares"), represented by depositary receipts ("uniQure DRs") issued to the AMT shareholders as consideration for the AMT Business. At the date of transfer, AMT had 31,101,065 issued shares.

On April 26, 2012, the distribution record date, AMT was placed in liquidation and made an initial distribution to shareholders of one uniQure DR for every AMT share held. Subsequently, AMT had no material assets, no further distributions were made to AMT shareholders and the liquidation process of AMT was completed in November 2012.

Following the transfer from AMT to uniQure, each AMT shareholder holding at least 5% of the shares in the capital of AMT on April 26, 2012, was entitled to exchange its uniQure DRs for an equal number of uniQure class A ordinary shares with a nominal value of one euro cent ("class A ordinary shares").

As part of the transaction with AMT, uniQure assumed a €5.0 million convertible loan provided to AMT by Forbion in December 2009. On April 5, 2012, this loan, together with accrued interest of €320,000, was converted into class A ordinary shares at a price of €1.00 per share, resulting in the issue to Forbion of 5.32 million class A ordinary shares. The terms of the conversion represented an amendment to the originally agreed conversion price of €3.69 per AMT share. This amendment to the conversion terms formed part of the terms of the combination of AMT and uniQure and the associated financing of uniQure, and was approved by AMT shareholders on March 31, 2012.

On April 5, 2012, uniQure raised €6.0 million through an issue to Forbion of 9,771,987 newly-issued class A ordinary shares at a price of €0.614 per share.

#### uniQure capital structure following the transactions on April 5, 2012

Following the transaction with AMT and the financing by Forbion, uniQure had a single class of shares. All shares were ordinary shares with the same economic rights in respect of dividends and upon a winding up or sale of the business. The ordinary shares were sub-divided into class A ordinary shares and class B ordinary shares. An additional classification of uniQure class C ordinary shares with a nominal value of one euro cent ("class C ordinary shares") was created on July 22, 2013. While the A, B and class C ordinary shares all have the same economic rights, the principal difference is that class A ordinary shares and class C ordinary shares are held directly by shareholders, whereas the class B ordinary shares are held by a trust foundation (*stichting administratiekantoor* (the "STAK")) on behalf of the uniQure DR holders; the STAK Trustees attend uniQure shareholder meetings on behalf of the uniQure DR holders and will follow voting instructions from the uniQure DR holders in respect of any resolutions at shareholder meetings.

#### Accounting for the formation of uniQure and the reorganization of the AMT Group

The basis of accounting for the combination of uniQure and the AMT Group was determined by International Financial Reporting Standard ("IFRS") 3, *Business Combinations*.

The AMT Group is the acquirer for accounting purposes and the transaction was accounted for as a reverse acquisition based on the following:

- The senior management of AMT became the senior management of uniQure;
- The former shareholders of AMT became shareholders in uniQure following the combination and exchange of AMT shares for uniQure shares, giving them 100% control and voting rights;
- The majority of the Supervisory Board members were independent and appointed by the former AMT shareholders;

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### Notes to Consolidated Financial Statements

- The AMT Group was significantly larger in size compared to uniQure based on the scale of activities and number of employees (uniQure had no employees); and
- uniQure issued equity interests to affect the business combination and was one of the combining entities that existed before the reorganization.

As a result, comparative figures as of and for the year ended December 31, 2011 are included in respect of the operations and financial position of the AMT Group in the consolidated financial statements of uniQure for 2012.

At the date of combination of uniQure and AMT on April 5, 2012, uniQure had €18,000 in cash as a consequence of the initial capital provided by Forbion on the creation of uniQure; this represented an initial payment towards the €6.0 million equity commitment by Forbion into the combined business on the basis described above. uniQure held no other assets and did not carry on any trading activities.

#### Restatement of AMT's 2011 consolidated financial statements and inclusion in uniQure's 2012 consolidated financial statements

The consolidated financial statements of AMT as of and for the year ended December 31, 2011 were prepared after the transaction with uniQure was complete and after AMT had been placed into liquidation.

AMT's consolidated financial statements for the year ended December 31, 2011 were originally prepared on the following basis:

- AMT, the parent company, was in liquidation; therefore, the related accounts were prepared on a liquidation basis rather than a going concern basis;
- At December 31, 2011 it was regarded as probable that the business and assets of AMT would be disposed of, and therefore the entire AMT Business was classified as assets and liabilities held for sale, and as discontinued operations; and
- At the date of preparation of the 2011 AMT consolidated financial statements it was known that the transaction between uniQure and AMT had been completed and that the AMT Business would continue as a going concern. Accordingly, there was no impairment provision against the book values and accordingly the change of basis from going concern to liquidation did not affect income or equity.

In preparing the financial statements for uniQure for 2012, uniQure included the AMT Group consolidated comparative financial information as of and for the year ended December 31, 2011, excluding the share capital of AMT. This information included was prepared on a going concern basis, rather than a liquidation basis, in order to be consistent and comparable for the periods disclosed.

This change in the basis of preparation does not result in any material adjustment to the equity or net income amounts disclosed in the 2011 AMT consolidated financial statements, although it does change the format of the presentation. Specifically, the AMT consolidated accounts for 2011 presented the AMT Business as discontinued activities, with assets and liabilities held for sale which are now presented in the uniQure 2012 consolidated financial statements on the basis that they formed part of the continuing operations of uniQure in 2011. See Note 5 below for further details.

#### Development of uniQure after April 5, 2012

Following completion of the transaction with AMT, uniQure focused on four of its remaining pre-clinical gene therapy programs (for the treatments of hemophilia B, acute intermittent porphyria, Sanfilippo B syndrome and Parkinson's disease).

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### Notes to Consolidated Financial Statements

On April 5, 2012, uniQure entered into the financing arrangements described above, consisting of the subscription for €6.0 million in new equity and the conversion of loans plus interest amounting to €5.32 million.

On April 19, 2012, uniQure raised a further €1.0 million through the issue of class A ordinary shares at a price of €0.614 per share to Cooperatieve Gilde Healthcare II U.A. ("Gilde"), an existing shareholder of uniQure.

Following a fourth review of uniQure's MAA, in July 2012 the CHMP recommended approval for the restricted population of LPLD patients with recurrent pancreatitis, subject to additional post-marketing studies for efficacy. The European Commission granted this approval in October 2012. Following the approval of Glybera under exceptional circumstances, uniQure has begun to expand in order to prepare for the product's commercial launch, as well as continuing to develop its other pipeline assets. uniQure began hiring additional staff and the number of employees increased from approximately 45 in early 2012 to approximately 67 by December 31, 2012. This growth continued during 2013. The additional hiring and related activities increased uniQure's cash outflows and the business needed to raise further funding.

In November 2012, uniQure entered into agreements to raise €0.55 million through the issuance of 899,514 uniQure DRs to employees and other persons at a price of €0.614 per uniQure DR. Of these, 445,777 uniQure DRs, representing approximately €0.27 million, were issued prior to December 31, 2012, and the remaining 453,737 uniQure DRs, representing approximately €0.28 million, were issued after December 31, 2012.

On December 17, 2012, uniQure entered into an agreement to raise €3.497 million through the issuance of a convertible loan, of which €1.498 million was drawn down in the year ended December 31, 2012 and the balance was drawn down after the period covered by these financial statements. The fair value element of the loan is disclosed on the balance sheet as €1.45 million. The principal terms at the date of the convertible loan agreement were that the loan bear interest at a rate of 8% per annum, has a maturity date of December 31, 2013 and is convertible at a discount of 5% to the next equity round (provided that the maximum conversion price would be €1.00 per share and that the 5% discount would not be applied if doing so would result in a conversion price lower than €0.614 per share). Because the convertible loan is a compound instrument including an embedded financial derivative which is not closely related to the host contract, under IFRS the embedded derivative has been split out and accounted for separately. Further details of the loan terms, and of its recognition as a financial liability and an equity instrument, are set out in Note 12 below. The loan also entitled the lenders to warrants, further details of which are set out in Note 12 below. The terms of this loan and the accompanying warrants were amended on March 17, 2013 as part of the increase in the loan amount to €13.497 million as described further below in this Note 1.

#### Negative equity position at December 31, 2012 and December 31, 2011

As of December 31, 2012 and 2011, uniQure had a negative net equity position and low cash balances. Nevertheless, investors have continued to support the business and during 2013 the financial position of the business has improved significantly (further details of events since December 31, 2012 are described below).

The financial statements are therefore prepared on a going concern basis as described in this Note 1 above.

**UNIQURE B.V.****Notes to Consolidated Financial Statements****Events since December 31, 2012**

Since the end of the period covered by these financial statements, uniQure has entered into a number of significant transactions. These are described as Post Balance Sheet Events. These events are further described as follows:

On March 21, 2013, the terms of the December 17, 2012 convertible loan were amended and the amount of the loan was increased to €13.497 million through the provision of an additional €10.0 million convertible loan by a new investor, Collier International Partners V-A, L.P. ("Collier Capital").

On April 29, 2013, uniQure entered into three agreements with Chiesi which consisted of:

- (i) a commercialization agreement, under which uniQure granted Chiesi the exclusive rights to commercialize Glybera in Europe and other specified countries, specifically excluding the United States and Japan;
- (ii) a co-development and license agreement for the joint co-development by Chiesi and uniQure of uniQure's Hemophilia B gene therapy program, and the exclusive rights for Chiesi thereafter to commercialize in Europe and other specified countries, specifically excluding the United States, China and Japan;
- (iii) a subscription agreement pursuant to which Chiesi agreed to purchase 5,546,070 class C ordinary shares at a price of €2.52 per share for a total of €14.0 million.

The conditions relating to these three agreements were satisfied and the agreements became effective on June 30, 2013. We received a €2.0 million upfront payment under the commercialization agreement, a €15.0 upfront payment under the co-development and license agreement and €14.0 million for the sale of class C ordinary shares under the subscription agreement; the subscription by Chiesi for the class C ordinary shares took place on July 24, 2013.

On June 13, 2013, uniQure entered into a loan agreement with Hercules Technology Growth Capital ("HTGC") under which uniQure drew down a loan of \$10.0 million.

On July 24, 2013, uniQure entered into a lease for new premises at Hartwell Avenue, Lexington MA, US through its newly incorporated, wholly owned subsidiary uniQure, Inc.

On July 26, 2013, uniQure exercised its rights under the convertible loan agreement to trigger conversion of the €13.497 million convertible loan into 6,681,678 class A ordinary shares.

On September 24, 2013, uniQure amended the terms of the HTGC loan entered into on June 14, 2013, reducing the costs of the loan in exchange for 185,873 warrants.

**Significant shareholders**

The Company's significant shareholders at the date of approval of these consolidated financial statements include:

- Advent Venture Partners
- Chiesi
- Collier Capital
- Forbion

**UNIQURE B.V.****Notes to Consolidated Financial Statements**

- Gilde
- Grupo Netco and affiliates
- Lupus Alpha PE Champions
- Omnes Capital (formerly Credit Agricole Private Equity)

**Other matters**

The Company's business is not subject to seasonal influences.

The financial statements were approved for issue by the Directors on October 25, 2013.

**2. Summary of Significant Accounting Policies****Introductory notes on the basis of preparation and presentation of the financial statements**

As described in Note 1 above, the combination of uniQure and the AMT Business was accounted for as a reverse acquisition under IFRS 3. Accordingly, uniQure's consolidated financial statements consolidate the financial results of the uniQure Group for the 12 months ended December 31, 2012 (including the results of AMT prior to its acquisition by uniQure).

In respect of comparative figures for the year ended December 31, 2011, uniQure included the consolidated financial statements of AMT, restated as described in Note 1 above. Because this restatement derives from a change in the basis of preparation of the 2011 consolidated financial statements, uniQure also discloses the opening consolidated balances for the AMT Group for 2011 and the audited consolidated balance sheet of AMT as of December 31, 2010 in addition to the balance sheets as of December 31, 2011 and 2012.

The further principal accounting policies applied in the preparation of these consolidated financial statements are set out below. These policies have been consistently applied to all the years presented, unless otherwise stated.

**2.1 Basis of Preparation**

The consolidated financial statements of uniQure have been prepared in accordance with International Financial Reporting Standards ("IFRS") as issued by the International Accounting Standards Board and as adopted by the European Union for the financial years 2012 and 2011.

The consolidated financial statements have been prepared under the historical cost convention, except for any derivative instruments, which are recorded at fair value through profit or loss.

The consolidated financial statements are presented in Euros except where otherwise indicated.

Under IFRS 3, the acquisition of the AMT Business by uniQure from AMT, which was completed on April 5, 2012, is accounted for as a reverse acquisition; therefore, the financial information is presented on a continuing basis for the AMT Business and uniQure. Under IFRS 3 uniQure is the legal parent of the AMT Business but is regarded as the accounting acquiree; conversely the AMT Group is the legal subsidiary but the accounting acquirer in the consolidated financial statements.

As of December 31, 2011, AMT was 100% owner and controller of two subsidiaries, Amsterdam Molecular Therapeutics (AMT) B.V. ("AMT BV") and Amsterdam Molecular Therapeutics (AMT) IP B.V. ("AMT IP"). AMT also controlled a pre-existing trust foundation which had historically been used in the period to

**UNIQUE B.V.****Notes to Consolidated Financial Statements**

December 31, 2009 for AMT staff incentive purposes (the "Stichting Participatie AMT"). These three entities were consolidated within the historical AMT consolidated financial statements.

In addition, on December 29, 2011, AMT BV incorporated seven new subsidiary companies with the objective of optimizing grants and other revenue opportunities. These companies did not commence operations until January 1, 2012 and did not have any material assets or liabilities as of December 31, 2011, but were also included within the AMT consolidated accounts. As of January 1, 2012, the employment of all uniQure's research and development staff was transferred to these new entities.

Following completion of the acquisition of the AMT Business by uniQure on April 5, 2012, the subsidiaries of AMT were transferred to uniQure BV and were renamed as follows:

<b>COMPANY NAME</b>	<b>FORMERLY KNOWN AS</b>
uniQure biopharma B.V.	Amsterdam Molecular Therapeutics (AMT) B.V.
uniQure IP B.V.	Amsterdam Molecular Therapeutics (AMT) IP B.V.
uniQure manufacturing B.V.	AMT manufacturing B.V.
uniQure Assay Development B.V.	AMT Assay Development B.V.
uniQure Research B.V.	AMT Research B.V.
uniQure non clinical B.V.	AMT non clinical B.V.
uniQure QA B.V.	AMT QA B.V.
uniQure Process Development B.V.	AMT Process Development B.V.
uniQure clinical B.V.	AMT clinical B.V.
stichting participatie AMT*	stichting participatie AMT*

\* Stichting participatie AMT is a Trust, not a company, but met the conditions for consolidation within uniQure's consolidated financial statements. Stichting participatie AMT was established to facilitate AMT's employee incentive schemes for the period up to 2010.

As described in Note 1 above, the AMT parent legal entity was not transferred to uniQure, and in November 2012, the listing of AMT's ordinary shares on Euronext Amsterdam was cancelled and the company was liquidated.

**2.2 Changes in accounting policy and disclosures**

The preparation of financial statements in conformity with IFRS requires the use of certain critical accounting estimates. It also requires management to exercise its judgment in the process of applying uniQure's accounting policies. The areas involving a higher degree of judgment or complexity or areas where assumptions and estimates are significant to the financial statements are disclosed in Note 4.

**(a) New and amended standards adopted by uniQure**

There are no IFRS or IFRIC interpretations that are effective for the financial year beginning on or after January 1, 2012 that would be expected to have a material impact on uniQure.

## UNIQUE B.V.

## Notes to Consolidated Financial Statements

**(b) New standards, amendments and interpretations issued but not effective for the financial year beginning January 1, 2012 and not early adopted**

A number of new standards and amendments to standards and interpretations are effective for annual periods beginning after January 1, 2012 and have not been applied in preparing these consolidated financial statements. None of these are expected to have a material effect on the consolidated financial statements of the Company.

- IFRS 9, 'Financial instruments', addresses the classification, measurement and recognition of financial assets and financial liabilities. IFRS 9 was issued in November 2009 and October 2010. It replaces the parts of IAS 39 that relate to the classification and measurement of financial instruments. IFRS 9 requires financial assets to be classified into two measurement categories: those measured as at fair value and those measured at amortised cost. The determination is made at initial recognition. The classification depends on the entity's business model for managing its financial instruments and the contractual cash flow characteristics of the instrument. For financial liabilities, the standard retains most of the IAS 39 requirements. The main change is that, in cases where the fair value option is taken for financial liabilities, the part of a fair value change due to an entity's own credit risk is recorded in other comprehensive income rather than the income statement, unless this creates an accounting mismatch. The group is yet to assess IFRS 9's full impact and intends to adopt IFRS 9 no later than the accounting period beginning on or after 1 January 2015. The Company will also consider the impact of the remaining phases of IFRS 9 when completed by the IASB.
- IFRS 10, 'Consolidated financial statements', builds on existing principles by identifying the concept of control as the determining factor in whether an entity should be included within the consolidated financial statements of the parent company. The standard provides additional guidance to assist in the determination of control where this is difficult to assess. The Company has assessed IFRS 10's full impact and adopted IFRS 10 on January 1, 2013. This standard will not have a material impact on the Company.
- IFRS 11, 'Joint arrangements', outlines the accounting by entities that jointly control an arrangement. Joint control involves the contractual agreed sharing of control and arrangements subject to joint control are classified as either a joint venture (representing a share of net assets and equity accounted) or a joint operation (representing rights to assets and obligations for liabilities, accounted for accordingly). The Company has assessed IFRS 11's full impact and adopted IFRS 11 on January 1, 2013. This standard will not have a material impact on the Company.
- IFRS 12, 'Disclosures of Interests in Other Entities', includes the disclosure requirements for all forms of interests in other entities, including joint arrangements, associates, special purpose vehicles and other off balance sheet vehicles. The Company has assessed IFRS 12's full impact and adopted IFRS 12 on January 1, 2013. This standard will not have a material impact on the Company.
- IFRS 13, 'Fair value measurement,' aims to improve consistency and reduce complexity by providing a precise definition of fair value and a single source of fair value measurement and disclosure requirements for use across IFRSs. The requirements, which are largely aligned between IFRSs and US GAAP, do not extend the use of fair value accounting but provide guidance on how it should be applied where its use is already required or permitted by other standards within IFRSs or US GAAP. The Company has assessed IFRS 13's full impact and adopted IFRS 13 on January 1, 2013. This standard will not have a material impact on the Company.

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### Notes to Consolidated Financial Statements

- IAS 19, 'Employee benefits', was amended in June 2011. The amendments outline the accounting requirements for employee benefits, including short-term benefits (e.g. wages and salaries, annual leave), post-employment benefits such as retirement benefits, other long-term benefits (e.g. long service leave) and termination benefits. The Company only participates in defined contribution plans; therefore, no impact is expected from this amendment.
- IFRIC 21, "Levies" was issued in May 2013. It addresses when an entity should recognize a liability to pay a government levy (other than income taxes). IFRIC 21 is an interpretation of IAS 37, Provisions, Contingent Liabilities and Contingent Assets. The interpretation is applicable for annual periods beginning on or after January 1, 2014. Early application is permitted. The Company has assessed IFRIC 21's full impact and adopted IFRIC 21 on January 1, 2013. This standard will not have a material impact on the Company.

The IASB has also issued Exposure Drafts in which significant changes on accounting and disclosures are proposed on topics such as lease accounting and revenue recognition. If the current proposals lead to new or amended standards, the changes could have a substantial impact on uniQure's financial statements in the coming years. The effective date of the revised standards is still under discussion.

#### 2.3 Consolidation

Subsidiaries are entities over which the Company has the power to govern the financial and operating policies generally accompanying a shareholding of more than 50% of the voting rights. The existence and effect of potential voting rights that are currently exercisable or convertible are considered when assessing whether the Company controls another entity. Subsidiaries are fully consolidated from the date on which control is transferred to the Company. They are de-consolidated from the date that control ceases.

Inter-company transactions, balances, income and expenses on transactions between uniQure companies are eliminated. Profits and losses resulting from inter-company transactions that are recognized in assets are also eliminated. Accounting policies of subsidiaries have been changed where necessary to ensure consistency with the policies adopted by the Company.

#### 2.4 Segment Reporting

Operating segments are identified on the basis of whether the allocation of resources and/or the assessment of performance of a particular component of uniQure's activities are regularly reviewed by uniQure's chief operating decision maker as a separate operating segment. By these criteria, the activities of uniQure are considered to be one segment, which comprises the discovery, development and commercialization of innovative gene therapies and the segmental analysis is the same as the analysis for uniQure as a whole. The Management Board is identified as the chief operating decision maker, and reviews the consolidated operating results regularly to make decisions about the resources and to assess overall performance.

#### 2.5 Foreign Currency Translation

##### (a) Functional and Presentation Currency

Items included in the financial statements of each of uniQure's entities are measured using the currency of the primary economic environment in which the entity operates ("the functional currency") which historically in all cases has been Euros. The consolidated financial statements are presented in Euros, which is the Company's functional and presentation currency.



**UNIQUE B.V.****Notes to Consolidated Financial Statements****(b) Transactions and Balances**

Foreign currency transactions are translated into the functional currency using the exchange rates prevailing at the dates of the transactions. Foreign exchange gains and losses resulting from the settlement of such transactions and from the translation at year-end exchange rates of monetary assets and liabilities denominated in foreign currencies are recognized in the income statement.

Foreign exchange gains and losses that relate to borrowings and cash and cash equivalents are presented within 'Finance income' or 'Finance costs' while all other foreign exchange gains and losses are presented within 'Other losses—net' on the Consolidated Statement of Comprehensive Income.

**2.6 Notes to the cash flow statement**

The cash flow statement has been prepared using the indirect method. The cash disclosed in the cash flow statement is comprised of cash and cash equivalents. Cash comprises cash on hand and demand deposits. Cash equivalents are short-term, highly liquid investments that are readily convertible to known amounts of cash and are subject to an insignificant risk of changes in value. Cash flows denominated in foreign currencies have been translated at the average exchange rates. Exchange differences, if any, affecting cash items are shown separately in the cash flow statement. Interest paid and received, dividends received and income tax are included in the cash from operating activities.

**Supplemental information relating to the cash flow statement**

The conversion of the €5.0 million convertible loan, together with accrued interest of €0.32m, amounting to €5.32 million in aggregate (described in Note 1 above) represented a non cash item. Further details are set out in Note 10 below.

**2.7 Intangible Assets****(a) Licenses**

Acquired patents have a definite useful life and are carried at cost less accumulated amortization and impairment losses. Amortization is calculated using the straight-line method to allocate the cost of licenses over their estimated useful lives (generally 20 years unless a license expires prior to that date). Amortization begins when an asset is available for use.

**(b) Research and Development**

Research expenditures are recognized as expenses as incurred. Costs incurred on development projects are recognized as intangible assets as of the date that it can be established that it is probable that future economic benefits that are attributable to the asset will flow to the Company considering its commercial and technological feasibility, generally when a filing is made for regulatory approval for commercial production, and when costs can be measured reliably. Given the current stage of the development of the Company's products and product candidates, no development expenditures have yet been capitalized. Registration costs for patents are part of the expenditures for a research and development project. Therefore, registration costs for patents are expensed as incurred as long as the applicable research and development project concerned does not yet meet the criteria for capitalization.

**UNIQUE B.V.****Notes to Consolidated Financial Statements****2.8 Property, Plant and Equipment**

Property, plant and equipment comprise mainly laboratory equipment, leasehold improvements, furniture and computer hardware/software. All property, plant and equipment are stated at historical cost less depreciation. Historical cost includes expenditures that are directly attributable to the acquisition of the items.

Subsequent costs are included in the asset's carrying amount or recognized as a separate asset, as appropriate, only when it is probable that future economic benefits associated with the item will flow to uniQure and the cost of the item can be measured reliably. All other repairs and maintenance charges are expensed in the period in which such charges are incurred.

Depreciation is calculated using the straight-line method to allocate the cost of the assets to their residual values over their estimated useful lives. Property, plant and equipment are depreciated as follows:

- Leasehold improvements periods between 5 - 15 years
- Laboratory equipment periods between 5 - 10 years
- Computer hardware/software 3 years

The assets' residual values and useful lives are reviewed, and adjusted if appropriate, at each balance sheet date. An asset's carrying amount is written down immediately to its recoverable amount if the asset's carrying amount is greater than its estimated recoverable amount (also refer to Note 7 below).

Gains and losses on disposals are determined by comparing proceeds with the carrying amount and are recognized in the income statement.

Operating leases and financial leases are described further in Note 2.21 below.

**2.9 Impairment of Non-Financial Assets**

Assets that are not subject to amortization (whether or not they are ready for use) are tested annually for impairment. Assets that are subject to amortization are reviewed for impairment whenever events or changes in circumstances indicate that the carrying amount may not be recoverable. An impairment loss is recognized for the amount by which the asset's carrying amount exceeds its recoverable amount.

The recoverable amount is the higher of an asset's fair value less costs to sell and value in use. For the purpose of assessing impairment, assets are grouped at the lowest levels for which there are separately identifiable cash flows (i.e. cash-generating units). Non-financial assets that have been previously impaired are reviewed for possible reversal of the impairment at each subsequent reporting date.

**2.10 Recognition and measurement**

Financial assets and financial liabilities are included in uniQure's balance sheet when uniQure becomes a party to the contractual provisions of the instrument. Financial assets are derecognized when the rights to receive cash flows from the investments have expired or have been transferred and the company has transferred substantially all risks and rewards of ownership.

**UNIQUE B.V.****Notes to Consolidated Financial Statements****Non-derivative financial instruments***Cash and cash equivalents*

Cash and cash equivalents includes bank balances, demand deposits and other short-term, highly liquid investments (with less than three months to maturity) that are readily convertible into a known amount of cash and are subject to an insignificant risk of fluctuations in value.

*Financial liabilities and equity*

Financial liabilities and equity instruments issued by uniQure are classified according to the substance of the contractual arrangements entered into and the definitions of a financial liability and an equity instrument. An equity instrument is any contract that evidences a residual interest in the assets of uniQure after deducting all its liabilities. The accounting policies adopted for specific financial liabilities and equity instruments are set out below.

*Trade payables*

Trade payables are obligations to pay for goods or services that have been acquired in the ordinary course of business from suppliers. Trade payables are classified as current liabilities if payment is due within one year or less (or in the normal operating cycle of the business if longer). If not, they are presented as non-current liabilities.

Trade payables are initially measured at fair value, and are subsequently measured at amortized cost, using the effective interest rate method.

*Equity instruments*

Equity instruments issued by uniQure are recorded at the proceeds received. Direct issuance costs are processed as a deduction on equity.

**Derivative financial instruments**

uniQure does not have a policy of engaging in speculative transactions, nor does it issue or hold financial instruments for trading purposes.

uniQure has entered into various financing arrangements with its investors, including convertible loans. These convertible loans each include embedded financial derivative elements (being the right to acquire equity in the Company at a future date for a pre-determined price). Therefore while uniQure does not engage in speculative trading of derivative financial instruments, it may hold such instruments from time to time as part of its financing arrangements.

Derivatives are initially recognized at fair value on the date a derivative contract is entered into and are subsequently re-measured at their fair value. The resulting gain or loss is recognized in the consolidated income statement, as the Company currently does not apply hedge accounting.

**2.11 Offsetting financial instruments**

Financial assets and liabilities are offset and the net amount is reported in the balance sheet when there is a legally enforceable right to offset the recognized amounts and there is an intention to settle on a net basis, or realize the asset and settle the liability simultaneously.

**UNIQUE B.V.****Notes to Consolidated Financial Statements****2.12 Equity and Borrowings**

The Company classifies an instrument, or its component parts, on initial recognition as a financial liability or an equity instrument in accordance with the substance of the contractual arrangement and the definitions of a financial liability and an equity instrument.

An instrument is classified as a financial liability when it is either (i) a contractual obligation to deliver cash or another financial asset to another entity; or (ii) a contract that will or may be settled in the Company's own equity instruments and is a non-derivative for which the Company is or may be obliged to deliver a variable number of the Company's own equity instruments or a derivative that will or may be settled other than by the exchange of a fixed amount of cash or another financial asset for a fixed number of the Company's own equity instruments.

An equity instrument is defined as any contract that evidences a residual interest in the assets of an entity after deducting all of its liabilities. An instrument is an equity instrument only if the issuer has an unconditional right to avoid settlement in cash or another financial asset.

**Ordinary Shares**

Ordinary shares are classified as equity. Incremental costs directly attributable to the issue of new shares or options are shown in equity as a deduction from the proceeds, net of tax.

**Convertible Loan**

Where the Company issues convertible loans that do not have the unconditional right to avoid delivering cash or a variable number of shares to settle obligations towards loan note holders, the Company accounts for such loan notes as containing an element that qualifies as a financial liability. Convertible loans are split into a debt component and a separate conversion option component. The conversion option is recognized initially at fair value, based on a probability-weighted scenario analysis. The debt component is the residual amount after deducting from the fair value of the loan as a whole (i.e. the issuance proceeds) the amount separately determined for the conversion option component. The debt component is subsequently carried at amortized cost using the effective interest rate method. When estimates regarding the amount or timing of payments required to settle the obligation change, the carrying amount of the financial liability is adjusted to reflect actual and revised estimated cash flows. The carrying amount is recalculated by computing the present value of estimated future cash flows at the financial instrument's original effective interest rate. Such adjustments are recognized as income or expense in the income statement. Any incremental costs of the loan are deducted from the carrying amount and are amortized over the term of the convertible loan under the effective interest rate method.

The conversion option is classified as a liability if it may be settled by either party other than by the exchange of a fixed amount of cash for a fixed number of the entity's own equity instruments. In that case, the conversion option is carried at fair value with changes in fair value recorded in the income statement. If the conversion option qualifies as an equity instrument, it is recognized in equity on issue date and not re-measured.

**2.13 Borrowings**

Borrowings are recognized initially at fair value, net of transaction costs incurred. Borrowings are subsequently carried at amortized cost. Any difference between the proceeds (net of transaction costs) and the redemption value is recognized in the income statement over the period of the borrowings using the effective interest rate method.

**2.14 Deferred Corporate Income Taxes**

There is no tax charge in the Company's Consolidated Statements of Comprehensive Income, nor any deferred tax recognized in the balance sheet for the periods covered by these financial statements.

**UNIQUIRE B.V.****Notes to Consolidated Financial Statements**

To the extent that any tax expense would arise, it would comprise current and deferred tax. Tax effects are recognized in the income statement, except to the extent that they relate to items recognized in other comprehensive income or directly in equity. In this case, the tax is also recognized in other comprehensive income or directly in equity, respectively.

The Company's management periodically evaluates positions taken in tax returns with respect to situations in which applicable tax regulation is subject to interpretation. It establishes provisions where appropriate on the basis of amounts expected to be paid to the tax authorities.

Deferred income tax is recognized, using the liability method, on temporary differences arising between the tax basis of assets and liabilities and their carrying amounts in the consolidated financial statements. However, the deferred income tax is not accounted for if it arises from initial recognition of an asset or liability in a transaction other than a reorganization that at the time of the transaction affects neither accounting nor taxable profit and loss. Deferred income tax is determined using tax rates (and laws) that have been enacted or substantially enacted by the balance sheet date and are expected to apply when the related deferred income tax asset is realized or the deferred income tax liability is settled.

Deferred income tax assets are recognized only to the extent that it is probable that future taxable profit will be available against which the temporary differences can be utilized.

Deferred income tax is provided on temporary differences arising on investments in subsidiaries and associates, except where the timing of the reversal of the temporary difference is controlled by the Company and it is probable that the temporary difference will not reverse in the foreseeable future.

Deferred income tax assets and liabilities are offset when there is a legally enforceable right to offset current tax assets against current tax liabilities and when the deferred income taxes assets and liabilities relate to income taxes levied by the same taxation authority on either the taxable entity or different taxable entities where there is an intention to settle the balances on a net basis.

**2.15 Employee Benefits****(a) Pension Obligations**

uniQure operates a defined contribution pension plan for all employees, which is funded by the Company through payments to an insurance company. uniQure has no legal or constructive obligation to pay further contributions if the plan does not hold sufficient assets to pay all employees the benefits relating to employee service in the current and prior periods. The contributions are recognized as employee benefit expense when they are due. Prepaid contributions are recognized as an asset to the extent that a cash refund or a reduction in the future payments is available.

**(b) Termination benefits**

Termination benefits are payable when employment is terminated by the Company before the normal retirement date, or whenever an employee accepts voluntary redundancy in exchange for these benefits. The Company recognizes termination benefits when it is demonstrably committed to either: terminating the employment of current employees according to a detailed formal plan without possibility of withdrawal; or providing termination benefits as a result of an offer made to encourage voluntary redundancy. Benefits falling due more than 12 months after the end of the reporting period are discounted to their present value.

**(c) Bonus plans**

The Company recognizes a liability and an expense for bonus plans if contractually obligated or if there is a past practice that has created a constructive obligation.

**UNIQUE B.V.****Notes to Consolidated Financial Statements****2.16 Share-Based Compensation****uniQure 2012 share option plan**

The Company operates a share-based payment plan, which is an equity settled share option plan under which options have been granted in 2012.

The fair value of the options in exchange for the services received is recognized as an expense. The total amount to be expensed over the vesting period, if any, is determined by reference to the fair value of the options granted. For the equity-settled option plan, the fair value is determined at the grant date. For share-based payments that do not vest until the employees have completed a specified period of service, uniQure recognizes the services received as the employees render service during that period. For the allocation of the expenses to be recognized, the Company treats each installment of a graded vesting award as a separate share option grant. The share options' vesting periods are as follows: 33.33% vests after one year from the initial vesting date and the remaining 66.66% vest daily on a straight-line pro rata basis over years two and three.

At each balance sheet date, the Company revises its estimates of the number of options that are expected to become exercisable. It recognizes the impact of the revision of original estimates, if any, in the income statement and a corresponding adjustment to equity.

**AMT 2010 option plan**

These consolidated financial statements include expenses relating to the equity-settled AMT share option plan ("AMT 2010 Plan"), which was operated prior to the transfer of the AMT Business to uniQure. Details of the cancellation of the AMT 2010 Plan and the related impact on the Company's consolidated financial statements are set out in Note 10 below.

**2.17 Provisions**

Provisions are recognized when uniQure has a present legal or constructive obligation as a result of past events, it is probable that an outflow of resources will be required to settle the obligation, and the amount can be reliably estimated.

Provisions are initially measured at fair value, and are subsequently measured at amortized cost, using the effective interest rate method.

**2.18 Revenues and Other Income**

Revenues comprise the fair value of the consideration received or receivable for the sale of licenses and services in the ordinary course of the Company's activities. Revenue is shown net of value-added tax, returns, rebates and discounts and after eliminating sales within the group.

The Company recognizes revenues when the amount of revenue can be reliably measured, it is probable that future economic benefits will flow to the entity and when specific criteria have been met for each of the Company's activities as described below. The Company bases its estimates on historical results, taking into consideration the type of customer, the type of transaction and the specifics of each arrangement.

License revenue can comprise upfront payments and milestone payments. uniQure's other income comprises certain subsidies that support uniQure's research efforts in defined research and development projects. These subsidies generally provide for reimbursement of approved costs incurred as defined in various grants.

Other income comprises grants, described further below in Note 2.19 below, as well as reimbursement of expenditure incurred under certain collaboration agreements.

**UNIQUE B.V.****Notes to Consolidated Financial Statements****2.19 Government grants**

The Company receives certain government and regional grants that support its research effort in defined projects. These grants generally provide for reimbursement of approved costs incurred as defined in the respective grants. Income in respect of grants includes contributions towards the costs of research and development. Income is recognized when costs under each grant are incurred in accordance with the terms and conditions of the grant and the collectability of the receivable is reasonably assured.

Government and regional grants relating to costs are deferred and recognized in the income statement over the period necessary to match them with the costs they are intended to compensate. When the cash in relation to recognized government or regional grants is not yet received the amount is included as a receivable on the balance sheet.

Where the grant income is directly related to the specific items of expenditure incurred, the income will be netted against such expenditure. Where the grant income is not a specific reimbursement of expenditure incurred, the Company includes such income under 'Other income' in the income statement.

Grants or investment credits may be repayable if uniQure successfully commercializes a relevant program that was funded in whole or in part by the grant or investment credit within a particular timeframe.

Prior to successful commercialization, uniQure does not make any provision for repayment.

**2.20 Recognition of research and development expenses**

Research expenditures are recognized as expenses when incurred except when certain criteria for capitalization as intangible assets are met (Note 2.7). At each balance sheet date, the Company estimates the level of service performed by the vendors and the associated cost incurred for the services performed.

**2.21 Leases****Operating leases**

Leases in which a significant portion of the risks and rewards of ownership are retained by the lessor are accounted for as operating leases. Payments made under operating leases (net of any incentives received from the lessor) are charged to the income statement on a straight-line basis over the period of the lease.

**Finance leases**

The Company leases certain laboratory equipment and office equipment. Leases for leasehold improvements and equipment where the Company bears substantially all the risks and rewards of ownership are accounted for as finance leases. Finance leases are capitalized at the lease's commencement at the lower of the fair value of the leased property or the present value of the minimum lease payments.

Each finance lease payment is allocated between the liability and finance charges in order to achieve a constant rate on the finance balance outstanding. The finance balances, net of finance charges, are included in other long-term payables. The interest element of the finance cost is charged to the income statement over the lease period to produce a constant periodic rate of interest on the remaining balance of the liability for each period. The laboratory and office equipment acquired under finance leases are depreciated over the shorter of the useful life of the asset or the lease term.

**2.22 Dividend Distributions**

Dividend distributions to the Company's shareholders are recognized as a liability in uniQure's financial statements in the period in which the dividends are approved by the Company's shareholders. To date uniQure has not, and AMT did not, pay dividends.

**UNIQURE B.V.****Notes to Consolidated Financial Statements****3. Financial Risk Management****3.1 Financial Risk Factors**

uniQure's activities have exposed it to a variety of financial risks: market risk (including currency risk, price risk, and cash flow and fair value interest rate risk), credit risk, and liquidity risk. uniQure's overall risk management program is focused on preservation of capital and the unpredictability of financial markets and has sought to minimize potential adverse effects on uniQure's financial performance and position.

Risk management is carried out by the finance department. The finance department identifies and evaluates financial risks and hedges these risks if deemed appropriate.

**(a) Market Risk****(i) Currency risk**

uniQure operates within the Euro area and also internationally and is exposed to foreign exchange risk arising from various currency exposures, primarily with respect to the U.S. dollar and, to a lesser extent, the British pound as the Company acquires certain materials and pays for certain licenses and other services in these two currencies.

In the years presented, uniQure had no significant outstanding receivables or payables in currencies other than Euros. Foreign exchange rate movements had no material effect on the financial statements presented.

In the absence of significant foreign exchange exposure, management has not set up a policy to manage the foreign exchange risk against the functional currency.

As of December 31, 2012 and December 31, 2011, there would not have been a significant effect on the Company's loss due to strengthening or weakening of the functional currency against any foreign currency.

**(ii) Price risk**

The market prices for the provision of preclinical and clinical materials and services, as well as external contracted research may vary over time. The commercial prices of any of the Company's products or product candidates are currently uncertain. The Company is not exposed to commodity price risk.

uniQure does not hold investments classified as available-for-sale or at fair value through profit or loss; therefore uniQure is not exposed to equity securities price risk.

**(iii) Cash flow and fair value interest rate risk**

The Company's interest rate risk arises from short and long-term borrowings. The Company has no borrowings with variable rates and is not exposed to cash flow interest rate risk. Borrowings issued at fixed rates expose the Company to fair value interest rate risk. During 2012 and 2011, the Company's borrowings were wholly denominated in Euros.

uniQure has neither significant long-term interest-bearing assets nor significant long-term interest bearing liabilities other than the €3,497,000 convertible loan described above, which was increased after the end of the period covered by these financial statements to €13,497,000 on March 21, 2013 and subsequently converted into 6,681,678 class A ordinary shares on July 26, 2013, as described in Note 1 above. uniQure does not enter into any interest rate swaps.



# UNIQUE B.V.

## Notes to Consolidated Financial Statements

### (b) Credit Risk

As described in Note 3(a) above, uniQure has no large receivable balances with external parties. As of December 31, 2012 and December 31, 2011, the majority of uniQure's cash and cash equivalents were placed at the following banks.

(€ in thousands)	AS OF DECEMBER 31,			
	2011	CREDIT RATING (MOODY'S)	2012	CREDIT RATING (MOODY'S)
	AMOUNT		AMOUNT	
<b>Bank</b>				
Rabo Bank	1,088	AAA	258	AA2
Van Lanschot	5	A-*	5	A-*
Deutsche Bank	7	A2	—	n/a
<b>Total</b>	<b>1,100</b>		<b>263</b>	

\* Rating is by Fitch

There are no financial assets past due date or impaired.

### (c) Liquidity Risk

Management considers uniQure's cash and cash equivalents as of December 31, 2012, when taken together with additional funds raised since that date (described further in Note 1 above), are sufficient to carry out the business plans going forward, at least until 12 months from the date of these financial statements. Prudent liquidity risk management implies maintaining sufficient cash, and planning to raise cash if and when needed, either through issue of shares or through credit facilities. Management monitors rolling forecasts of uniQure's liquidity reserve on the basis of expected cash flow.

The table below breaks down uniQure's financial liabilities into relevant maturity groups based on the remaining period at the balance sheet date to the contractual maturity date, including interest obligations arising during the relevant periods. The amounts disclosed in the table are the contractual undiscounted cash flows. Balances due within 12 months equal their carrying balances as the impact of discounting is not significant.

On December 17, 2012, the Company agreed to issue €3,497,000 in convertible loan notes to certain of its shareholders. The Company drew down €1,498,000 of the convertible loan in December 2012 which remained outstanding at December 31, 2012, was repayable within 12 months from December 31, 2012, and which carried interest at a rate of 8 percent per annum. Accordingly, at December 31, 2012 the Company had an contractual liability amounting to €1,618,000 (in respect of principal plus interest) falling due within 12 months in respect of the drawn down element of the convertible loan.

The Company drew down an additional €1,999,000 after the end of the period covered by these financial statements.

# UNIQUE B.V.

## Notes to Consolidated Financial Statements

Subsequent to year end on March 21, 2013, uniQure amended the terms of the 2012 convertible loan notes and issued an additional €10,000,000 in convertible loan notes to a new investor, Collier Capital.

	LESS THAN 1 YEAR	BETWEEN 1 AND 2 YEARS	BETWEEN 2 AND 5 YEARS	OVER 5 YEARS
	(€ in thousands)			
<b>December 31, 2012</b>				
Trade and other payables	4,067	450	—	—
Debt to related party	1,618	—	—	—
<b>Total</b>	<b>5,685</b>	<b>450</b>	<b>—</b>	<b>—</b>
<b>December 31, 2011</b>				
Trade and other payables	3,673	180	—	—
Debt to related party	250	250	5,250	—
<b>Total</b>	<b>3,923</b>	<b>430</b>	<b>5,250</b>	<b>—</b>

The financial instruments by category are as follows:

DECEMBER 31, 2012				
	LOANS AND RECEIVABLES	ASSETS AT FAIR VALUE THROUGH PROFIT AND LOSS	DERIVATIVES USED FOR HEDGING	AVAILABLE FOR SALE
	(€ in thousands)			
<b>Assets as per balance sheet</b>				
Trade and other receivables	841	—	—	—
Financial assets at fair value through profit and loss	—	—	—	—
Cash and cash equivalents	263	—	—	—
<b>Total</b>	<b>1,104</b>	<b>—</b>	<b>—</b>	<b>—</b>

	LIABILITIES AT FAIR VALUE THROUGH PROFIT AND LOSS	DERIVATIVES USED FOR HEDGING	OTHER FINANCIAL LIABILITIES AT AMORTIZED COST	TOTAL
	(€ in thousands)			
<b>Liabilities as per balance sheet</b>				
Debt to related party	132	—	1,366	1,498
Finance lease liabilities	—	—	601	601
Trade and other payables	—	—	3,916	3,916
<b>Total</b>	<b>132</b>	<b>—</b>	<b>5,883</b>	<b>6,015</b>

DECEMBER 31, 2011				
	LOANS AND RECEIVABLES	ASSETS AT FAIR VALUE THROUGH PROFIT AND LOSS	DERIVATIVES USED FOR HEDGING	AVAILABLE FOR SALE
	(€ in thousands)			
<b>Assets as per balance sheet</b>				
Trade and other receivables	1,084	—	—	—
Cash and cash equivalents	1,100	—	—	—
<b>Total</b>	<b>2,184</b>	<b>—</b>	<b>—</b>	<b>—</b>

	LIABILITIES AT FAIR VALUE THROUGH PROFIT AND LOSS	DERIVATIVES USED FOR HEDGING	OTHER FINANCIAL LIABILITIES AT AMORTIZED COST	TOTAL
	(€ in thousands)			
<b>Liabilities as per balance sheet</b>				
Debt to related party	2	—	4,542	4,544
Finance lease liabilities	—	—	221	221
Trade and other payables	—	—	3,632	3,632
<b>Total</b>	<b>2</b>	<b>—</b>	<b>8,395</b>	<b>8,397</b>

**UNIQUE B.V.****Notes to Consolidated Financial Statements****3.2 Capital Risk Management**

The Company's objectives when managing capital are to safeguard the Company's ability to continue as a going concern in order to provide returns for shareholders, benefits for other stakeholders and to maintain an optimal capital structure to reduce the cost of capital.

In order to maintain or adjust the capital structure, the Company may adjust the amount of dividends paid to shareholders (although at this time the Company does not have retained earnings and is therefore currently unable to pay dividends), return capital to shareholders, issue new shares or sell assets to reduce debt.

The total amount of equity as recorded on the balance sheet is managed as capital by the Company.

For financial instruments that are measured on the balance sheet at fair value, IFRS 7 requires disclosure of fair value measurements by level of the following fair value measurement hierarchy:

- Quoted prices (unadjusted) in active markets for identical assets or liabilities (level 1);
- Inputs other than quoted prices included within level 1 that are observable for the asset or liability, either directly (that is, as prices) or indirectly (that is, derived from prices) (level 2);
- Inputs for the asset or liability that are not based on observable market data (that is, unobservable inputs) (level 3).

As of December 31, 2012 and 2011 financial instruments at fair value through profit and loss amounted to €(464,000) and €207,000, respectively, and comprised of movements on the fair value of the derivative elements of convertible loans, as described further in Note 17 below.

The fair value of financial instruments that are not traded in an active market (for example, over-the-counter derivatives) is determined by using valuation techniques. These valuation techniques maximize the use of observable market data where it is available and rely as little as possible on entity specific estimates. If all significant inputs required to fair value an instrument are observable, the instrument is included in level 2.

If one or more of the significant inputs is not based on observable market data, the instrument is included in level 3.

The Company's assets and liabilities that are measured at fair value at December 31, 2012 and 2011 are all measured as level 2 financial instruments. The carrying amount of a financial asset or financial liability is a reasonable approximation of the fair value and therefore information about the fair values of each class has not been disclosed.

**4. Critical Accounting Estimates and Judgments**

Estimates and judgments are continually evaluated and are based on historical experience and other factors, including expectations of future events that are believed to be reasonable under the circumstances.

**4.1 Critical accounting estimates and assumptions**

The Company makes estimates and assumptions concerning the future. The resulting accounting estimates will, by definition, seldom equal the related actual results. The estimates and assumptions that have a significant risk of causing a material adjustment to the carrying amounts of assets and liabilities within the next financial year are addressed below.

**UNIQURE B.V.****Notes to Consolidated Financial Statements****Share-based payments**

In 2012 the Company introduced an equity settled share option plan. At the balance sheet date 8,031,777 options were outstanding (2011: 1,898,200 options relating to the AMT share option plan). This plan is accounted for in accordance with the policy as stated in note 2.16. The option pricing model used and the inputs to that model are described in Note 10 below.

**Corporate taxes**

The Company is subject to corporate taxes in the Netherlands. Significant judgment is required in determining the use of net operating loss carry forwards and taxation of upfront and milestone payments for corporate tax purposes. There are many transactions and calculations for which the ultimate tax determination is uncertain. Where the final tax outcome of these matters is different from the amounts that were initially recorded, such differences will impact the current and deferred corporate tax assets and liabilities in the period in which such determination is made.

**Research and development expenditures**

Research and development expenditures are not capitalized but are reflected in the income statement because the criteria for capitalization are not met (note 4.2). As of each balance sheet date, the Company estimates the level of service performed by its vendors or other counterparties and the associated costs incurred for the services performed.

Although the Company does not expect its estimates to be materially different from amounts actually incurred, its understanding of the status and timing of services performed relative to the actual status and timing of services performed may vary and could result in reporting amounts that are too high or too low in any particular period.

**4.2 Critical judgments in applying the entity's accounting policies****(a) Corporate Income Taxes**

The Dutch corporate income tax act permits reporting pursuant to a consolidated tax regime, referred to as a fiscal unity. A fiscal unity is a combination of a parent and subsidiaries whereby formally the parent, in our case uniQure B.V., is the entity that is taxed for the consolidated profits of the fiscal unity.

uniQure, which has a history of tax losses, recognizes deferred tax assets arising from unused tax losses or tax credits only to the extent that the relevant fiscal unity has sufficient taxable temporary differences or there is convincing other evidence that sufficient taxable profit will be available against which the unused tax losses or unused tax credits can be utilized by the fiscal unity. Management's judgment is that sufficient convincing other evidence is not available and a deferred tax asset is therefore not recognized.

**(b) Research and Development Expenditures**

The stage of a particular project generally forms the basis for the decision whether costs incurred for the Company's research and development projects can be capitalized or not. In general, the Company's vision is that clinical development expenditures are not capitalized until the Company files for regulatory approval in respect of the program, as this is considered to be the first point in time when it becomes probable that future revenues can be generated. However, although the EMA has now granted marketing authorization under exceptional circumstances in the EU for Glybera, such authorization is subject to further conditions before first sales may be made in the EU.

IAS38 describes the conditions under which development expenditure should be capitalized. These conditions include the availability of adequate technical, financial and other resources to complete the development of the intangible asset. At December 31, 2012 uniQure lacked the financial and other

**UNIQUE B.V.****Notes to Consolidated Financial Statements**

resources required to complete the remaining development of Glybera to meet the EMA conditions. On this basis the conditions described in IAS 38 were not met and accordingly no development expenditure amounts were capitalized.

As of each balance sheet date, the Company estimates the level of service performed by its vendors or other counterparties and the associated costs incurred for the services performed. As part of the process of preparing the Company's financial statements the Company is required to estimate its accrued expenses. This process involves reviewing quotations and contracts, identifying services that have been performed on the Company's behalf, estimating the level of service performed and the associated cost incurred for the service when it has not yet been invoiced or otherwise notified of the actual cost. The majority of the Company's service providers invoice the Company monthly in arrears for services performed or when contractual milestones are met. The Company makes estimates of its accrued expenses as of each balance sheet date in its financial statements based on facts and circumstances known to it at that time. The Company periodically confirms the accuracy of its estimates with the service providers and makes adjustments if necessary. The significant estimates in its accrued research and development costs are related to fees paid to clinical research organizations, or CROs, in connection with research and development activities for which the Company has not yet been invoiced. The Company bases its expenses related to CROs on its estimates of the services received and efforts expended pursuant to quotes and contracts with CROs that conduct research and development on its behalf.

The financial terms of these agreements are subject to negotiation, vary from contract to contract and may result in uneven payment flows. There may be instances in which payments made to vendors and other counterparties will exceed the level of services provided and result in a prepayment of the research and development costs. In accruing service fees, the Company estimates the time period over which services will be performed and the level of effort to be expended in each period. If the actual timing of the performance of services or the level of effort varies from the Company's estimate, it adjusts the accrual or prepayment expense accordingly. Although the Company does not expect its estimates to be materially different from amounts actually incurred, its understanding of the status and timing of services performed relative to the actual status and timing of services performed may vary and could result in reporting amounts that are too high or too low in any particular period.

**(c) Impairment of Assets**

Assets that are subject to amortization are reviewed for impairment whenever events or changes in circumstances indicate that the carrying amount may not be recoverable. In the year ended December 31, 2012, management reviewed the carrying amount of these assets and determined that no adjustments to carrying values were required.

In the year ended December 31, 2011, management recorded an impairment charge of €300,000 in respect of the termination of a research license under which uniQure had made an initial payment of €300,000; this payment had been determined as an intangible asset, and accordingly this amount has been written off. Management determined that no further impairment charges were required in respect of the 2011 consolidated financial statements.

The Company tests assets that are not subject to amortization annually for impairment. For the purpose of assessing impairment, the Company groups assets at the lowest levels for which there are separately identifiable cash flows (cash-generating units). The Company currently uses all material assets in the development of certain gene therapy products. Therefore, the management regularly reviews all activities of the Company as a single component and one cash-generating unit. Although we are not currently selling any products, our collaborator, Chiesi, is preparing the commercial launch of Glybera in the EU. The Company's future revenues from product sales, if any, will depend on the success of Chiesi's commercialization efforts and the Company's success in obtaining marketing authorization for Glybera and any other product candidates in additional countries.

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**Notes to Consolidated Financial Statements**

The Company has determined that no impairment should be recorded during the year ended December 31, 2012. Based on management's expectations of revenues and gross margin from anticipated sales of Glybera by Chiesi, the management has determined that no impairment charge in respect of intangible assets relating to Glybera is necessary. These expectations are principally based on management's estimate of the market size for Glybera and the gross margin that management expects to realize.

**(d) Compound Financial Instruments**

Management classifies a financial instrument or its component parts on initial recognition as a financial liability, a financial asset or an equity instrument in accordance with the substance of the contractual arrangement and the definitions of a financial liability, a financial asset and an equity instrument. As described under Note 12, we have analyzed the convertible loan issued in 2012 and concluded that both the loan and the convertible elements qualified as financial liabilities; Note 12 contains further details relating to the valuation of the convertible element.

**5. Reconciliation of 2011 comparative financial information**

As described in Note 1 above, the consolidated financial statements of uniQure for the year ended December 31, 2012 include the restated AMT Group consolidated comparative financial information for the year ended December 31, 2011.

The restatement of the consolidated financial statements of AMT for the year ended December 31, 2011 resulted in the following adjustments.

UNIQUE B.V.

Notes to Consolidated Financial Statements

Assets and liabilities

	AT DECEMBER 31, 2011		2011 RESTATED FOR UNIQUE CONSOLIDATED ACCOUNTS
	PER AMT CONSOLIDATED 2011 AUDITED ACCOUNTS	ADJUSTMENT (€ in thousands)	
<b>Assets</b>			
<b>Non-current assets</b>			
Intangible assets	—	2,725	2,725
Property, plant and equipment	—	895	895
<b>Non-current assets</b>	<b>—</b>	<b>3,620</b>	<b>3,620</b>
<b>Current assets</b>			
Receivables from related parties	—	35	35
Social security and other taxes	—	249	249
Other receivables	—	800	800
Cash and cash equivalents	—	1,100	1,100
Assets held for sale	5,804	(5,804)	—
<b>Current assets</b>	<b>5,804</b>	<b>(3,620)</b>	<b>2,184</b>
<b>Total assets</b>	<b>5,804</b>	<b>—</b>	<b>5,804</b>
<b>Liabilities</b>			
<b>Non-current liabilities</b>			
Financial lease liabilities	—	180	180
Debt to related party	—	4,544	4,544
	<b>—</b>	<b>4,724</b>	<b>4,724</b>
<b>Current liabilities</b>			
Trade payables	—	1,736	1,736
Social security and other taxes	—	713	713
Debt to related party	—	—	—
Other current liabilities	—	1,224	1,224
Liabilities held for sale	8,397	(8,397)	—
	<b>8,397</b>	<b>(4,724)</b>	<b>3,673</b>
<b>Total liabilities</b>	<b>8,397</b>	<b>—</b>	<b>8,397</b>

# UNIQUE B.V.

## Notes to Consolidated Financial Statements

### Result for the period

	FOR THE YEAR ENDED DECEMBER 31, 2011		
	PER AMT CONSOLIDATED 2011 AUDITED ACCOUNTS	ADJUSTMENT	2011 RESTATED FOR UNIQUE CONSOLIDATED ACCOUNTS
	(€ in thousands, except share data and per share data)		
Other income	—	2,192	2,192
<b>Total operating profit</b>	—	<b>2,192</b>	<b>2,192</b>
Research and development costs	—	(15,500)	(15,500)
General and administrative costs	—	(3,781)	(3,781)
Other losses—net	—	(26)	(26)
<b>Total operating costs</b>	—	<b>(19,038)</b>	<b>(19,038)</b>
Operating result	—	(17,116)	(17,116)
Finance income	—	277	277
Finance costs	—	(462)	(462)
<b>Result before corporate income taxes</b>	—	<b>(17,300)</b>	<b>(17,300)</b>
Corporate income taxes	—	—	—
Result for the year from continuing operations	—	(17,300)	(17,300)
Result for the year from discontinued operations	(17,300)	17,300	—
<b>Result for the year</b>	<b>(17,300)</b>	<b>—</b>	<b>(17,300)</b>
<b>Attributable to:</b>			
<b>Ordinary shareholders of the Company</b>	<b>(17,300)</b>	<b>—</b>	<b>(17,300)</b>
<b>Loss per share for result attributable to the equity holders of the Company during the year</b>			
Basic and diluted loss per share from continuing operations	—	(0.73)	(0.73)
Basic and diluted loss per share from discontinued operations	(0.73)	0.73	—
<b>Basic and diluted loss per share</b>	<b>(0.73)</b>	<b>—</b>	<b>(0.73)</b>



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Notes to Consolidated Financial Statements

Consolidated Statement of Changes in Equity

	PER AMT CONSOLIDATED 2011 AUDITED ACCOUNTS	ADJUSTMENT (€ in thousands)	2011 RESTATED FOR UNIQUE CONSOLIDATED ACCOUNTS
<b>Balance at December 31, 2010</b>			
Share capital	940	(705)	235
Share premium	99,136	705	99,841
<b>Total</b>	<b>100,076</b>	<b>—</b>	<b>100,076</b>
<b>Capital contributions</b>			
Share capital	10	(8)	2
Share premium	98	8	106
<b>Total</b>	<b>108</b>	<b>—</b>	<b>108</b>
<b>Balance at December 31, 2011</b>			
Share capital	950	(713)	237
Share premium	99,234	713	99,947
<b>Total</b>	<b>100,184</b>	<b>—</b>	<b>100,184</b>

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Notes to Consolidated Financial Statements

Cash flow for the period

	PER AMT CONSOLIDATED 2011 AUDITED ACCOUNTS	ADJUSTMENT (€ in thousands)	2011 RESTATED FOR UNIQUE CONSOLIDATED ACCOUNTS
<b>Cash flow from operating activities</b>			
Result before corporate income tax	—	(17,300)	(17,300)
adjustments for:	—		
—Depreciation	—	600	600
—Impairment of assets	—	300	300
—Derivative result	—	(207)	(207)
—Exchange result	—	26	26
—Share-based payment expenses	—	940	940
—Changes in working capital	—	(1,427)	(1,427)
—Interest (income)/expense	—	365	365
Cash used in operations	—	(16,703)	(16,703)
Interest paid	—	(2)	(2)
<b>Net cash used in continuing operating activities</b>	<b>—</b>	<b>(16,705)</b>	<b>(16,705)</b>
<b>Net cash used in discontinued operating activities</b>	<b>(16,705)</b>	<b>16,705</b>	
<b>Net cash used in operating activities</b>	<b>(16,705)</b>	<b>—</b>	<b>(16,705)</b>
<b>Cash flow from investing activities</b>			
Purchases of property, plant and equipment		(200)	(200)
Purchases of intangible assets		(109)	(109)
Interest received		147	147
<b>Net cash used in continuing investing activities</b>		<b>(162)</b>	<b>(162)</b>
<b>Net cash used in discontinued investing activities</b>	<b>(162)</b>	<b>162</b>	<b>—</b>
<b>Net cash used in investing activities</b>	<b>(162)</b>	<b>—</b>	<b>(162)</b>
Capital contribution from shareholders			108
<b>Net cash generated from continuing financing activities</b>	<b>—</b>	<b>108</b>	<b>108</b>
<b>Net cash generated from discontinued financing activities</b>	<b>108</b>	<b>(108)</b>	<b>—</b>
<b>Net cash generated from financing activities</b>	<b>108</b>	<b>—</b>	<b>108</b>
<b>Net decrease in cash, cash equivalents and other bank overdrafts of continuing activities</b>	<b>—</b>	<b>(16,759)</b>	<b>(16,759)</b>
<b>Net decrease in cash, cash equivalents and other bank overdrafts of discontinued activities</b>	<b>(16,759)</b>	<b>16,759</b>	<b>—</b>
<b>Net decrease in cash, cash equivalents and other bank overdrafts of continuing and discontinued activities</b>	<b>(16,759)</b>	<b>—</b>	<b>(16,759)</b>
Cash, cash equivalents and bank overdrafts at the beginning of the year	17,859	—	17,859
<b>Cash, cash equivalents at the end of the year (not classified as assets held for sale)</b>	<b>—</b>	<b>1,100</b>	<b>1,100</b>
<b>Cash, cash equivalents at the end of the year (classified as assets held for sale)</b>	<b>1,100</b>	<b>(1,100)</b>	<b>—</b>
<b>Cash, cash equivalents at the end of the year</b>	<b>1,100</b>	<b>—</b>	<b>1,100</b>

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Notes to Consolidated Financial Statements

6. Intangible Assets

(€ in thousands)	<u>LICENSES</u>
<b>At January 1, 2011</b>	
Cost	3,216
Accumulated amortization and impairment	(300)
<b>Net book amount</b>	<b>2,916</b>
<b>Year ended December 31, 2011</b>	
Opening net book amount	2,916
Additions	109
Amortization and impairment charge	(300)
<b>Closing net book amount</b>	<b>2,725</b>
<b>At December 31, 2011</b>	
Cost	3,025
Accumulated amortization and impairment	(300)
<b>Net book amount</b>	<b>2,725</b>
<b>Year ended December 31, 2012</b>	
Opening net book amount	2,725
Additions	553
Amortization and impairment charge	—
<b>Closing net book amount</b>	<b>3,278</b>
<b>At December 31, 2012</b>	
Cost	3,278
Accumulated amortization and impairment	—
<b>Net book amount</b>	<b>3,278</b>

In the years presented in these financial statements, no amortization expense was recorded because the related products for which licenses have been granted have either not yet been approved for commercial sale by regulatory authorities or, at the date of these financial statements, uniQure lacked the financial and technical resources to be confident of completing the remaining development, and therefore such approved products are not yet available for use. Since no amortization expense was recognized during the year, management estimated the recoverable amount of these licenses at the end of each annual reporting period although there was no indication that the licenses may be impaired.

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The net book amount of uniQure's intangible assets by licensor is set out below:

(€ in thousands)	AS OF DECEMBER 31,	
	2011	2012
Xenon	210	365
AmpliPhi	2,198	2,352
NIH	317	317
UCSF	—	244
<b>Total</b>	<b>2,725</b>	<b>3,278</b>

The amounts set out above arose as follows:

In June 2001, the Group obtained a sub-license from Xenon Genetics, Inc. ("Xenon"), which was approved by Xenon's licensor, The University of British Columbia. The sub-license was initially capitalized in the amount of €140,000. Xenon granted the Group the exclusive worldwide rights to use the Xenon licensed technology and to use, manufacture, distribute and sell licensed products (as defined in the sub-license agreement). The contract provides for payment of license fees, milestone payments, and a portion of the royalties received from Chiesi, which will be payable to Xenon instead. Dependent upon the progress and success of the research and development activities and sales by the Company, future milestones are capitalized when payment is probable. In 2006, the Company paid a milestone of €70,000 that was capitalized.

In December 2006, the Group acquired a sub-license from Targeted Genetics Corporation (now renamed AmpliPhi Biosciences, Inc. ("AmpliPhi")). The sub-license was approved by AmpliPhi's licensor, The University of Pennsylvania. It is related to "AAV1 Vector" technology, and the recognized acquisition amount is €1,330,000, which was capitalized.

In 2007, the Group acquired a license from the National Institutes of Health ("NIH") in the amount of €208,000 for the production of adeno-associated virus vectors.

In 2008, the Company paid and capitalized a milestone payment of €357,000 to AmpliPhi under the above license.

In 2008, the Group capitalized licensing fees totaling €600,000 related to a license from the La Sapienza University of Rome ("La Sapienza") for technology for treatment for Duchenne Muscular Dystrophy and a license from the San Raffaele University of Milano for technology to be used in the treatment of Factor IX Hemophilia.

In 2009, the Group accrued for and capitalized a licensing milestone of \$750,000 (€511,000) to AmpliPhi which became payable on the submission of the MAA of Glybera to EMA. The payment to AmpliPhi was made in 2010.

In 2010, the Group terminated its research and license agreement with San Raffaele University of Milano. This expense had been capitalized as an intangible asset, and accordingly this amount has been written off.

In 2011, the Group made and capitalized a payment to the NIH in the amount of €109,000 for a license to use adeno-associated virus serotype 5.

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**Notes to Consolidated Financial Statements**

During 2011, the Group stopped further development of its Duchenne Muscular Dystrophy program. At that time, the program had not met its scientific goals. Accordingly, the amount capitalized as an intangible asset in respect of the license from La Sapienza described above has been written off.

In 2012, the Group made and capitalized a payment to AmpliPhi Biosciences Corporation of \$200,000 (€154,000) in accordance with its financial obligations relating to Glybera.

In 2012, the Group also made and capitalized a payment to Xenon of CAN\$ 200,000 (€155,000) in respect of Glybera's approval by EMA.

In 2012, the Group made and capitalized a payment to the University of California at San Francisco ("UCSF") of \$300,000 (€244,000) in respect of the license to certain data, know-how, and other rights relating to the program for Parkinson's disease.

In the year ended December 31, 2012, uniQure did not capitalize any development expenses related to Glybera for the period following the approval of the MAA for Glybera because at that time uniQure lacked the financial and technical resources to be confident of completing the remaining development.

Management determined that based on its expectations of revenues and gross margin following market launch, no other impairment charge is necessary.

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## Notes to Consolidated Financial Statements

### 7. Property, Plant and Equipment

	LEASEHOLD IMPROVEMENT	LABORATORY EQUIPMENT	COMPUTER HARDWARE/ SOFTWARE	TOTAL
	(€ in thousands)			
<b>As of January 1, 2011</b>				
Cost	721	2,839	504	4,064
Accumulated amortization and impairment	(385)	(1,963)	(430)	(2,778)
<b>Net book amount</b>	<b>336</b>	<b>876</b>	<b>74</b>	<b>1,286</b>
<b>Year ended December 31, 2011</b>				
Opening net book amount	336	876	74	1,286
Additions	49	100	51	200
Depreciation charge	(123)	(414)	(54)	(591)
<b>Closing net book amount</b>	<b>262</b>	<b>562</b>	<b>71</b>	<b>895</b>
<b>As of December 31, 2011</b>				
Cost	770	2,939	555	4,264
Accumulated amortization and impairment	(508)	(2,377)	(484)	(3,369)
<b>Net book amount</b>	<b>262</b>	<b>562</b>	<b>71</b>	<b>895</b>
<b>Year ended December 31, 2012</b>				
Opening net book amount	262	562	71	895
Additions	494	20	324	838
Depreciation charge	(158)	(312)	(78)	(548)
<b>Closing net book amount</b>	<b>598</b>	<b>270</b>	<b>317</b>	<b>1,185</b>
<b>As of December 31, 2012</b>				
Cost	1,264	2,959	879	5,102
Accumulated amortization and impairment	(666)	(2,689)	(562)	(3,917)
<b>Net book amount</b>	<b>598</b>	<b>270</b>	<b>317</b>	<b>1,185</b>

#### Closing net book amount

Leasehold improvements include a net book value as of December 31, 2012 of €396,000 (2011: €nil) where uniQure is lessee under a finance lease. A further description of financial lease contracts is set out in Note 11 below. A further description is set out in Note 2.8 above.

Following the reorganization in 2011, uniQure entered into revised rental agreements with AMC and its representatives, as a consequence of which certain parts of the premises, with a cost of €446,000 at December 31, 2012, are now accounted for under a finance lease instead of an operating lease; the assets covered by this change in contractual arrangements are included within the amount of €494,000 shown as additions to leasehold improvements for the year ended December 31, 2012.

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## Notes to Consolidated Financial Statements

### 8. Trade and Other Receivables

(€ in thousands)	2011	2012
<b>Receivables from related parties (Note 24)</b>	<b>35</b>	<b>26</b>
VAT to be received	249	418
Tax on wages to be received	—	—
Social Security to be received	—	—
<b>Total taxes and social securities</b>	<b>249</b>	<b>418</b>
Accounts receivable	2	0
Interest to be received	121	2
Prepaid expenses	—	—
Other receivables	677	395
<b>Other receivables and prepayments</b>	<b>800</b>	<b>397</b>

The fair value of trade and other receivables approximates their carrying value. As of December 31, 2012 and 2011, all trade or other receivables were assessed as fully recoverable. The carrying amount of the Company's trade receivables are fully denominated in Euros.

The other classes within trade and other receivables do not contain impaired assets. The maximum exposure to credit risk at the reporting date is the carrying value of each class of receivable mentioned above. The Company does not hold any collateral as security.

### 9. Cash and Cash Equivalents

(€ in thousands)	2011	2012
Cash at bank and in hand	694	263
Short-term bank deposits	406	—
	<b>1,100</b>	<b>263</b>

The effective interest rate on short-term bank deposits was 1.5% in the year ended December 31, 2012 (1.5% in the year ended December 31, 2011); these deposits have an average maturity of 1 day.

### 10. Shareholders' Equity

uniQure was incorporated on January 10, 2012; therefore, the year ending December 31, 2012 is the first accounting period for the Company. As described in Note 1 above, the business combination between uniQure and the AMT Group is accounted for as a reverse acquisition and the consolidated financial statements of the AMT Business are presented as the consolidated financial statements of uniQure, with an adjustment required to reflect the capital of uniQure.

The amount recognized as issued equity interests in the consolidated financial statements is determined by the issued equity interest in AMT outstanding immediately prior to the business combination, but the equity structure (the number and type of equity interests issued) reflects the equity structure of uniQure.

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## Notes to Consolidated Financial Statements

Accordingly the share capital and share premium accounts of AMT disclosed in its audited consolidated financial statements for prior years are restated as if uniQure ordinary shares had been issued. The exchange ratio of uniQure shares issued for AMT shares was 1-for-1, but because AMT shares had a nominal value of €0.04 and uniQure shares have a nominal value of €0.01, the impact of this approach is to reduce the balance of the share capital reported within the previous AMT accounts and correspondingly increase the balance on the share premium account.

	NUMBER OF SHARES	AMOUNT OF AMT CAPITAL (BASED ON SHARES OF €0.04 NOMINAL VALUE) (€ in thousands)	AMOUNT OF UNIQUE CAPITAL (BASED ON SHARES OF €0.01 NOMINAL VALUE)
<b>Share capital (ordinary shares)</b>			
<b>As of January 1, 2011</b>	<b>23,512,225</b>		
Share capital		940	235
Share premium		99,136	99,841
<b>Total</b>		<b>100,076</b>	<b>100,076</b>
<b>New shares issued</b>	<b>235,902</b>		
Share capital		10	2
Share premium		98	106
<b>Total</b>		<b>108</b>	<b>108</b>
<b>As of December 31, 2011</b>	<b>23,748,127</b>		
Share capital		950	237
Share premium		99,234	99,947
<b>Total</b>		<b>100,184</b>	<b>100,184</b>
<b>New shares issued prior to April 5, 2012</b>	<b>7,352,938</b>		
Share capital		294	74
Share premium		2,206	2,426
<b>Total</b>		<b>2,500</b>	<b>2,500</b>
<b>Shares in issue at April 5, 2012</b>	<b>31,101,065</b>		
Share capital		1,244	311
Share premium		101,440	102,373
<b>Total</b>		<b>102,684</b>	<b>102,684</b>
<b>New shares issued after April 5, 2012</b>	<b>17,166,428</b>		
Share capital		n/a	172
Share premium		n/a	12,422
<b>Total</b>		<b>n/a</b>	<b>12,594</b>
<b>As of December 31, 2012</b>	<b>48,267,493</b>		
Share capital		n/a	483
Share premium		n/a	114,795
<b>Total</b>		<b>n/a</b>	<b>115,278</b>

During the period covered by these financial statements, uniQure had a single class of shares, which are denominated as ordinary shares. Within this class of ordinary shares, there are further sub-denominations between class A ordinary shares and class B ordinary shares. Other than the fact that certain corporate



**UNIQUE B.V.****Notes to Consolidated Financial Statements**

resolutions require the approval of the general meeting of the class A ordinary shares, class A ordinary shares and class B ordinary shares carry equal economic rights and rank equally.

Following the general meeting of shareholders of uniQure on July 22, 2013, the Company's authorized share capital was increased from €1,900,000 or 190,000,000 shares, to €2,000,000 or 200,000,000 shares through the creation of an additional €100,000 or 10,000,000 class C ordinary shares, in connection with the intended equity investment by Chiesi which took place on July 24, 2013. The authorized share capital of uniQure is as follows:

	<b>A</b>	<b>B</b>	<b>C</b>	<b>TOTAL</b>
Number of Ordinary Shares	171,406,311	18,593,689	10,000,000	200,000,000
Value (€)	1,714,063	185,937	100,000	2,000,000

As of December 31, 2012, a total of 48,267,493 shares were issued and paid up in full at a nominal value of €0.01 per share (2011: 23,748,127 AMT shares at €0.04 per share prior to adjustment in accordance with IFRS 3 and restated as if they were uniQure shares at €0.01 per share). Of these, 24,512,366 are presented as being issued during the year (2011: 235,902 shares). The total gross payment with respect to these shares issued during the period is presented as €15,094,000 (2011: €108,000).

Note 1 describes the shares issued during the period since January 1, 2012. In summary these were as follows:

- On January 4, 2012, AMT raised €2,500,000 through the issuance of 7,352,938 new shares at a price of €0.34 per share. On April 5, 2012, uniQure acquired the AMT Business, issuing 31,101,665 class B ordinary shares, represented by uniQure DRs to the AMT Shareholders as consideration. Since this transaction is accounted for as a reverse acquisition, this issue of uniQure DRs is not disclosed separately within the consolidated financial record of the business;
- On April 5, 2012, uniQure raised €6,000,000 through the issue of 9,771,987 class A ordinary shares to Forbion, at a price of €0.614 per share. On April 5, 2012, the Company issued 5,320,000 class A ordinary shares to Forbion, at a price of €1.00 per share in consideration of the conversion of the outstanding €5,000,000 in convertible loan notes, together with accrued interest of €320,000;
- On May 17, 2012, uniQure raised €1,000,000 through the issue of 1,628,664 class A ordinary shares to Gilde, at a price of €0.614 per share; and
- In November and December 2012, pursuant to an agreement entered into in April 2012, the Company raised a total amount of €274,000 through the issuance of an aggregate of 445,777 class B ordinary shares, represented by uniQure DRs shares to employees and related parties at a price of €0.614 per share.

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## Notes to Consolidated Financial Statements

	NARRATIVE (SEE NOTE 1)	CASH ITEMS	NON CASH ITEMS	TOTAL
		(€ in thousands)		
Jan 4, 2012	Investment in AMT ordinary shares	2,500	—	2,500
Apr 5, 2012	Forbion new equity investment	6,000	—	6,000
Apr 5, 2012	Forbion conversion of existing convertible loan plus interest	—	5,320	5,320
Apr 19, 2012	Gilde new equity investment	1,000	—	1,000
Nov-Dec, 2012	Employees and other persons new equity investment	274	—	274
		<b>9,774</b>	<b>5,320</b>	<b>15,094</b>

In 2012 and 2011, no new shares were issued upon the exercise of share options. On December 31, 2012, 36,294 shares were held by the stichting participatie AMT as treasury shares (2011: 36,294). (Further details of stichting participatie AMT are set out in Note 2 above.) These treasury shares arose under the terms of an employee incentive plan operated by AMT, under which employees were permitted to subscribe for new shares at a discount to the market price, but were then required to remain with AMT for a period of three years following the effective date of such purchase. Employees who left AMT within such three year period and who did not meet certain other exceptional conditions were obliged to return their shares.

### Share Premium

The presentation of the share premium account is on a consistent basis with the share capital account, including similar adjustments to reflect the impact of the treatment under IFRS 3, as set out in the table above.

The total additions to share premium in the year ended December 31, 2012 amount to €14,849,000 net of costs. This increase in share premium was due to the issue of shares as described above.

### Other Reserves

The costs of equity-settled share-based payments to employees are recognized in the income statement, together with a corresponding increase in equity during the vesting period, taking into account (deferral of) corporate income taxes. The accumulated expense of the share incentive plan recognized in the income statement is shown separately in the equity category Other Reserves in the Consolidated Statement of Changes in Equity. Accumulated expense related to the AMT share option plan (described further below) for the period up to April 5, 2012, amounting to €2,987,000, is offset against the retained losses at April 5, 2012 following the extinguishing of AMT and the AMT share option scheme, as set out in the Consolidated Statement of Changes in Equity.

In the years presented in these financial statements, the Company did not have any legal or other types of restricted reserves.

### Share Options

#### 2012 Share Option Plan

At the general meeting of shareholders on February 15, 2012, uniQure shareholders approved the adoption of the 2012 Plan. Under the 2012 Plan, share options are granted on the date of grant and vest over a period of three years on the basis set out in Note 2.16 above.

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## Notes to Consolidated Financial Statements

Any options that vest must be exercised by the tenth anniversary of the effective date of grant.

In 2012, 8,031,777 options were granted under the 2012 Plan to management and certain other employees and consultants. The expense recognized amounted to €1,508,000 during the year ended December 31, 2012.

On October 25, 2011, AMT announced a reorganization resulting in a reduction of the AMT Group's workforce of approximately 50% and subsequent transfer of its assets and liabilities to uniQure pursuant to the transaction entered into on April 5, 2012. Consequently, AMT's 2010 Plan was deemed to have been closed and the outstanding options thereunder cancelled. Accordingly, AMT recognized the remaining option expense for AMT 2010 Plan participants that remained with the Company following the reorganization on the basis of a reduced vesting period, and recognized the pro rata element of this charge in 2011. The consequence of this was a total option expense recognized and accounted for within retained earnings of €259,000 for the period January 1—April 5, 2012 (for the year ended December 31, 2011 the recognized charge amounted to: €940,000). On April 5, 2012, the AMT 2010 Plan and the outstanding options granted under it were cancelled. Accordingly, the accumulated reserve was transferred to retained earnings, as described in the Consolidated Statement of Changes in Equity above. Details regarding the granting of options under the AMT 2010 Plan are disclosed for comparative purposes, since the costs associated with this plan are included in the results for the year ended December 31, 2011.

Both the 2012 Plan and AMT 2010 Plan qualify as equity-settled plans. Movements in the number of outstanding share options granted in 2012 under the 2012 Plan and under the AMT 2010 Plan, all of which were granted in 2010 and 2011, were as follows:

	2011		2012	
	NUMBER	EXERCISE PRICE	NUMBER	EXERCISE PRICE
<b>Number of options outstanding as of January 1</b>	<b>1,354,150</b>	<b>1.95 - 2.92</b>	<b>1,898,200</b>	<b>1.95 - 2.92</b>
Number of options granted	751,207	2.06	8,031,777	0.614
Number of options lapsed	(269,550)	2.06 - 2.92	1,898,200	1.95 - 2.92
<b>Number of options outstanding as of December 31</b>	<b>1,898,200</b>	<b>1.95 - 2.92</b>	<b>8,031,777</b>	<b>0.614</b>

Of the 8,031,777 options outstanding (2011: 1,898,200), no options (2011: none) were exercisable. Options outstanding at the end of the year have the following weighted average remaining contractual life and ranges of exercise prices:

YEAR ENDED DECEMBER 31, 2012	RANGE EXERCISE	
WEIGHTED AVERAGE REMAINING CONTRACTUAL LIFE	PRICE	OPTIONS
	IN EUR PER SHARE	
1 - 5 years	—	—
6 years	—	—
7 years	—	—
8 years	—	—
9 years	0.614	8,031,777
At December 31, 2012	0.614	8,031,777

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Notes to Consolidated Financial Statements

YEAR ENDED DECEMBER 31, 2011	RANGE EXERCISE PRICE	
WEIGHTED AVERAGE REMAINING CONTRACTUAL LIFE	IN EUR PER SHARE	OPTIONS
1 - 5 years	—	—
6 years	—	—
7 years	—	—
8 years	1.95 - 2.92	1,113,250
9 years	2.06 - 2.92	784,950
<b>At December 31, 2011</b>	<b>1.95 - 2.92</b>	<b>1,898,200</b>

The Black-Scholes option pricing model has been used to value these awards, based on the following key variables:

	2012	2011
Options with change of control and service based vesting conditions	—	1,898,200
Options with an IPO, change of control and service based vesting conditions	8,031,777	—
Share Price: the closing share price on the grant dates	€0.614 - 1.02	€1.95 - 2.97
Expected Volatility: uniQure used an estimated volatility figure which was fixed based on volatility analysis of companies in the same sector and of a similar size	70 - 80%	50%
Expected Term: is the period from grant until the expected exercise date.	5.5 - 6.3 years	6 - 7 years
Exercise price (in €):	€0.614	€1.95 - 2.97
Expected Dividend Yield: the Company currently does not pay dividends and has no plans to do so	0%	0%
Risk-free Rate: based on Government bonds with a term commensurate with the expected term of each option tranche. Also considered is the risk-free rate over the performance period for each option tranche	0.5 - 1.1%	2.3%

Of the 8,031,777 options granted in 2012, 2,391,085 options were granted to members of the Management Board and 984,564 options were granted to members of the Supervisory Board.

All options granted in 2012 vest upon a liquidity event such as a change of control of the Company or an initial public offering ("IPO"). The total expense to be recognized under an IPO scenario, representing the uncharged part of the total fair value of these options remaining at December 31, 2012, approximates to €2.1 million (equivalent to an uncharged amount remaining of €0.26/option), which will be recognized over the vesting period.

**UNIQUE B.V.****Notes to Consolidated Financial Statements****Expected option term**

uniQure has considered various approaches to take into account the effects of expected early exercise whereby the length of the vesting period, the expected share price development, the expected share price volatility and the participants' employee level within the organization have been analyzed.

Based on the outcome of this analysis, uniQure management has determined to take the effects of expected early exercise into account by using an estimate of an option's expected life as an input into the Black-Scholes option pricing model. As historical data about employees' exercise behavior is not available, management's estimate is based on a weighted average expected option life for the entire participant group. The resulting expected weighted average life of the options granted is the midway between the vesting date and the contractual term of the options.

**Valuation of ordinary shares**

AMT shares were previously listed on Euronext Amsterdam. The initial valuation of €0.614 per uniQure share derived from the average closing price of AMT shares on each of the 5 business days immediately prior to February 17, 2012, the date of the announcement of the transaction between uniQure and AMT, which was also €0.614 per AMT share. Given that uniQure had no other business of its own, and that the consideration for purchase of the business and assets of AMT was a one-for-one share issue to AMT in respect of each AMT share then in issue, the company believed this value was reasonable and reflected the market valuation of the business.

At the date of each grant of options subsequent to the transaction between uniQure and AMT, the fair value of the ordinary shares is determined by the Management Board and Supervisory Board, and takes into account the most recently available valuation of ordinary shares and the assessment of additional objective and subjective factors the company believes are relevant.

**Expected volatility**

Prior to the transaction between uniQure and AMT on April 5, 2012, AMT was listed on the Euronext Amsterdam exchange from June 2007 through April 2012. This period has provided company-specific historical and implied volatility information. In April 2012, the weighting assigned to the company-specific historic volatility was 50%, and uniQure has also estimated the expected volatility based on the historical volatility of the publicly traded peer companies for the remaining 50% weighting. For option grants post April 2012, the volatility has been estimated solely by reference to the historical volatility of the publicly traded peer companies. This has resulted in a volatility in the range 70 - 80% in respect of the options granted in the year ended December 31, 2012.

Further details regarding the total expense recognized in the income statement for share options granted to managing directors, supervisory directors and selected employees are set out in Note 24. The corresponding increase in equity is separately accounted for as other reserves.

**11. Financial Lease Liabilities**

uniQure leases certain leasehold improvements by means of finance leases including the following:

- Agreement between Beheersmaatschappij Dienstverlening en Deelneming AZUA BV ("BDDA") a 100% subsidiary of the AMC, and uniQure regarding leasehold improvements at Meibergdreef 61, Amsterdam, from October 2005 for 11 years. The rent of the leasehold improvements amounts to €30,000 per year. The Company has the right to cancel the lease earlier on a one-year term; however, the Company will then need to repay the remaining amount of leased leasehold improvements.

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### Notes to Consolidated Financial Statements

- Agreement between BDDA and uniQure regarding leasehold improvements Meibergdreef 57, Amsterdam, from July 2006 for 10 years and three months. The rent of the leasehold improvements amounts to €23,000 per year. On July 1, 2012, AMC and uniQure amended the finance leases to include additional finance lease assets. As a result, at December 31, 2012, the financial lease liability amounted to €601,000 (2011: €221,000).

Finance lease liabilities are effectively secured as the rights to the leased asset revert to the lessor in the event of default. The carrying amount corresponds to the fair value as terms of the contracts were agreed at arm's length and market conditions for such contracts have not subsequently changed. The interest rate imposed by the lessor for all finance lease liabilities is 8% per annum.

	2011	2012
	(€ in thousands)	
Gross finance lease liabilities—minimum lease payments		
No later than 1 year	53	184
Later than 1 year and no later than 5 years	205	505
Later than 5 years	—	—
Future finance charges on finance leases	(37)	(88)
<b>Total</b>	<b>221</b>	<b>601</b>

Present value of finance lease liabilities

The present value of finance lease liabilities is as follows:

	2011	2012
	(€ in thousands)	
No later than 1 year	41	151
Later than 1 year and no later than 5 years	180	450
Later than 5 years	—	—
Future finance charges on finance leases	—	—
<b>Total</b>	<b>221</b>	<b>601</b>

## 12. Debt to related party

The change in Debt to related party in 2012 compared to 2011 reflects the conversion of the 2009 convertible loan in 2012 and the drawdown of a new convertible loan in December 2012, as described in Note 1 above.

### December 2012 Convertible loan

On December 17, 2012, uniQure entered into a convertible loan agreement with four of its major shareholders (Forbion, Gilde, Grupo Netco and Lupus Alpha), in respect of unsecured and unsubordinated loan notes, which have an issue price of 100% and pay an annual coupon of 8%. Of the total loan, €1,498,000 was drawn down at December 31, 2012 and the balance of €1,999,000 was drawn down in the period from January 1, 2013 to January 31, 2013. If converted, the notes would convert into class A ordinary shares of uniQure at a conversion price to be determined by certain factors but limited to a

**UNIQURE B.V.****Notes to Consolidated Financial Statements**

maximum conversion price of €1.00 per class A ordinary share. The conversion price could be adjusted if certain dilutive events occurred, including an issuance of shares at a discount to the average share price over the preceding five days. The loan noteholders were also issued warrants entitling them to invest an additional amount equivalent to up to 25% of their loan in class A ordinary shares at the conversion price, within a period of up to 5 years from the date of the loan or December 17, 2017. In March 2013, uniQure increased the loan by an additional €10,000,000, invested by Collier Capital. As part of the increase, the loan note terms for all loan note holders described in this paragraph were amended such that the final maturity date of the loan notes was extended to December 31, 2014. Additionally, the warrant entitlement was reduced to 10% of the principal amount of the loan provided to uniQure.

Upon recognition, the fair value of the liability portion of the December 2012 convertible loan is €1,366,000 and is included within the Current liabilities: Debt to related party—financial liability, on the Consolidated Balance Sheet as of December 31, 2012.

The conversion feature included in this loan agreement is not closely related to the host contract and therefore has been split and accounted for separately as a financial derivative measured at fair value through profit or loss. The fair value of this embedded derivative is € 132,000 and is included within the Current liabilities: Debt to related party—embedded derivative on the Consolidated Balance Sheet as of December 31, 2012.

**December 2009 Convertible loan**

On December 16, 2009, AMT entered into a convertible loan agreement with Forbion, one of its major shareholders, in respect of five-year unsecured and unsubordinated loan notes ("2009 Notes"), which had an issue price of 100% and paid an annual coupon of 5%. This loan was drawn down on December 23, 2009. During the conversion period, which started six months after the funding date (or at the earlier occurrence of a limited number of events, such as a public offer to acquire AMT) and which ended on the final maturity date, the 2009 Notes were convertible into ordinary shares of AMT at an initial conversion price of € 3.91, representing a conversion premium compared to AMT's share price at the date of issue of approximately 30%. The conversion price could be adjusted in the case of certain dilutive events, including an issue of shares at a discount to the average share price over the preceding five day period. As a consequence, the private placement by AMT on October 6, 2010, resulted in such an adjustment to the conversion price of the bonds from € 3.91 per share to € 3.69 per share, representing a conversion premium compared to AMT's share price at this date of 54%.

On April 5, 2012 the obligations under the loan were transferred from AMT to uniQure, and were then converted into new uniQure shares at a conversion price of € 1.00/share.

Further details on the accounting policy applied to the convertible loan agreement are described in paragraph 2.12 (convertible loan) above.

At December 31, 2011 the conversion price of the convertible loan was above the market price of AMT ordinary shares. In such a situation the convertible loan was not regarded as being dilutive at December 31, 2011.

The valuation methodology used for the option part employed a Black-Scholes approach on the assumption that the loan would not be converted before its maturity date.

## UNIQUE B.V.

### Notes to Consolidated Financial Statements

Under IFRS 7.27, the relevant factors considered within the valuation model for the compound of the instrument are as follows:

- AMT share price of €0.365 at December 31, 2011;
- Conversion price of €3.69 at December 31, 2011;
- Expected life of the instrument of 3 years.
- Annualized volatility of AMT share price of 50%;
- Implied call price of €5.535 (being 150% of the €3.69 exercise price)
- Annual rate of quarterly dividends of 0%; and
- Discount rate—Bond yield equivalent of 0.779%.

The rate used in 2011 for discounting the financial liability represented by the loan element of the convertible in 2011 was 8.5% per annum.

On February 17, 2012, AMT announced the sale and transfer of the AMT Business to uniQure. Under the terms of the transaction, the convertible loan was transferred to uniQure and then converted at a subscription price of €1.00 per share.

	2011	2012
	(€ in thousands)	
Loan component against amortized costs	4,542	1,366
Fair value of conversion right—embedded derivative	2	132
	<b>4,544</b>	<b>1,498</b>

### 13. Trade and Other Payables

Trade and other payables are as follows:

	2011	2012
	(€ in thousands)	
<b>Trade payables</b>	<b>1,736</b>	<b>2,099</b>
<b>Payables to related parties</b>	<b>—</b>	<b>1,366</b>
Wage taxes	653	130
Accrued social security costs	60	21
<b>Social security and other taxes</b>	<b>713</b>	<b>152</b>
Short-term lease liabilities	41	151
Accrued expenses	833	1,204
Other amounts to be paid	350	461
<b>Other current liabilities</b>	<b>1,224</b>	<b>1,816</b>

The carrying values of trade and other payables are assumed to approximate their fair values.



**UNIQUE B.V.****Notes to Consolidated Financial Statements****Other liabilities**

Other liabilities mainly consist of accruals for services provided by vendors but not yet billed, reimbursements received from research and development partners for expenses which have yet to be incurred and miscellaneous liabilities.

**14. Revenues and Other Income**

uniQure's other income consists of government subsidies and grants that support uniQure's research efforts in defined research and development projects.

Other income was €649,000 in 2012 (2011: €2,192,000).

Grant income was reduced in 2012 because the reorganization reduced uniQure's resources available to apply for and carry out work supported by such grants. In addition, Other income includes an element of rebate on payroll taxes; in 2012 the levels of rebate were reduced, and the level of payroll taxes paid by uniQure was also reduced following the reduction in headcount as a result of the reorganization.

**15. Expenses by Category**

Research and development costs amounted to €10,231,000 and €15,500,000 in 2012 and 2011, respectively, and consist of allocated employee costs, Good Manufacturing Practices ("GMP") facility costs, clinical development costs, collaboration costs, license costs, the costs of laboratory consumables and allocated depreciation costs. General and administrative costs amounted to €4,564,000 and €3,807,000 in 2012 and 2011, respectively, and consist of allocated employee costs, office costs, consultancy costs and administrative costs. Research and development costs and general administrative costs included the following costs by function:

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	<u>2011</u>	<u>2012</u>
	<u>(€ in thousands)</u>	
Employee benefit expenses (See note 16)	8,492	8,350
Laboratory and development expenses	4,854	2,065
Legal and advisory expenses	2,416	1,622
Office and housing expenses	1,420	1,197
Patents and licenses	853	619
Other operating expenses	683	394
Depreciation expenses (See note 7)	590	548
Other losses—net (exchange differences)	26	45
	<u>19,334</u>	<u>14,840</u>

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For leases where uniQure is a lessee under operating leases, lease rentals amounting to €393,000 (2011: €435,000) are included in "general and administrative costs" in the income statement.

# UNIQUE B.V.

## Notes to Consolidated Financial Statements

### 16. Employee Benefits

Wages and salaries in 2011 included termination expenses amounting to €228,000 incurred in respect of the redundancies of certain staff pursuant to the Company's restructuring.

	2011	2012
	(€ in thousands)	(€ in thousands)
Wages and salaries	5,499	4,553
Social security costs	502	361
Share options and depository receipts granted to directors and employees (See note 10)	940	1,767
Pension costs—defined contribution plans	400	303
Other employee expenses	1,151	1,366
	<b>8,492</b>	<b>8,350</b>
Number of employees at the end of the period	85	67

### 17. Finance Income and Cost

	2011	2012
	(€ in thousands)	(€ in thousands)
Finance income:		
Interest income current accounts	70	22
Derivative result	207	—
	<b>277</b>	<b>22</b>
Finance expense:		
Bank borrowings-overdrafts and other debt	(42)	—
Derivative result arising on early conversion of the loan	—	(464)
Loan from related party	(379)	(63)
Finance leases	(14)	(20)
	<b>(435)</b>	<b>(547)</b>
<b>Finance costs—net</b>	<b>(158)</b>	<b>(525)</b>

### 18. Income Tax Expense

(€ in thousands)	2011	2012
Current tax	—	—
Deferred tax	—	—
Profit/(loss) before tax	(17,300)	(14,716)
Expenses not deductible for tax purposes	741	2,268
Tax losses for which no deferred income tax asset was recognized	(16,559)	(12,448)
<b>Tax charge</b>	<b>—</b>	<b>—</b>

**UNIQUE B.V.****Notes to Consolidated Financial Statements**

No tax charges or liabilities were incurred in the years 2012 and 2011 since the Company was in a loss-making position. No deferred tax asset has been recognized in respect of carry-forward losses.

Under Dutch income tax law a tax loss carry-forward is subject to a time limitation of nine years. Losses incurred in the years up to 2004 can still be offset against profits up to and including 2013. In connection with the transfer of the AMT Business from AMT to uniQure, uniQure has discussed with Belastingdienst, the Dutch tax authorities, the transfer of all accumulated tax losses that relate to the AMT Business, excluding tax losses relating specifically to the activities of the AMT legal entity.

In order to promote innovative technology development activities and investments in new technologies, a corporate income tax incentive has been introduced in Dutch tax law called the Innovations Box. For the qualifying profits, the company effectively owes only 5% income tax, instead of the general tax rate of 25.5%. Because uniQure is loss-making it has not currently made any application to the tax authorities for such an agreement, but intends to do so when it reaches profitability.

uniQure has recognized the full amount of its losses in the year in which they were incurred. As noted above, these losses are available for use within nine years of being incurred. The total amount of tax losses carried forward was €106,274,000 as of December 31, 2012 (2011: €93,826,000).

The expiration dates of these losses is summarized in the following table. In the year ended December 31, 2012, the amount of unused tax losses that expired was € nil (2011: €644,000).

(€ in thousands)	2013	2014	2015	2016	2017	2018	2019	2020	2021
Loss expiring	56	1,336	1,838	3,310	35,633	16,735	18,359	16,559	12,448

**19. Earnings per Share****Basic Loss per Share**

(€ in thousands, except for per share data)	2011	2012
Result attributable to equity holders of the Company	(17,300)	(14,716)
Weighted average number of ordinary shares ('000)	23,549	43,187
<b>Basic loss per share</b>	<b>(0.73)</b>	<b>(0.34)</b>

Basic loss per share is calculated by dividing the profit attributable to equity holders of the Company by the weighted average number of shares outstanding during the period.

**Diluted Loss per Share**

Diluted loss per share is calculated by adjusting the weighted average number of ordinary shares outstanding to assume conversion of all dilutive potential ordinary shares. Due to the fact that the Company is loss making for the periods included in these financial statements, neither share options nor the convertible loan described above are included in the diluted earnings per share calculation. Consequently basic and diluted earnings per share are the same.

**UNIQURE B.V.****Notes to Consolidated Financial Statements****20. Dividends per Share**

The Company did not declare dividends for the years ended December 31, 2012 and December 31, 2011.

**21. Cash Flow Statement**

In the cash flow statement, proceeds from issuance of shares comprise:

(€ in thousands)	2011	2012
Issue of share capital	108	9,774
Expenses incurred and paid	—	—
	<b>108</b>	<b>9,774</b>

Further details relating to the issue of shares other than for cash are set out in Note 10 above, in relation to the issue of 5,320,000 class A ordinary shares to Forbion on April 5, 2012 on conversion of the convertible loan and accrued interest amounting to €5,320,000 in aggregate.

**22. Contingencies****Royalties and Milestones**

In the course of its business uniQure enters as a licensee into contracts with other parties with regard to the development and marketing of its pipeline products. Among other payment obligations, is obligated to pay royalties to the licensors based on future sales levels and milestone payments whenever defined milestones are met. As both future sales levels and the timing and achievement of milestones are uncertain, the financial effect of these agreements cannot be estimated reliably.

**23. Commitments****Operating Lease Commitments**

uniQure leases various office space and laboratory space under operating lease agreements. The Company leases its headquarters facilities under an agreement between uniQure and AMC, represented by BDDA and Amsterdam Vector Productions B.V. ("AVP"), both subsidiaries of AMC (Second Rental Agreement) in respect of facilities located at Meibergdreef 61 Amsterdam, from October 1, 2005 until September 30, 2016, and an agreement for the lease of facilities at Meibergdreef 57, Amsterdam, from July 1, 2006 until September 30, 2016. The aggregate annual lease payments amount to €360,000.

The lease expenditure charged to the income statement for operating leases amounts to €542,000 in the year ended December 31, 2012 (2011: €435,000). The future aggregate minimum lease payments under non-cancellable operating leases are as follows:

(€ in thousands)	2011	2012
No later than 1 year	435	542
Later than 1 year and no later than 5 years	1,632	1,627
Later than 5 years	—	—
	<b>2,067</b>	<b>2,169</b>

**UNIQUE B.V.****Notes to Consolidated Financial Statements****Research and Development Commitments**

uniQure has entered into research and development commitments in relation to uniQure's product pipeline. The future aggregate minimum payments under these research and development commitments are as follows:

(€ in thousands)	2011	2012
No later than 1 year	343	277
Later than 1 year and no later than 5 years	—	—
Later than 5 years	—	—
	<b>343</b>	<b>277</b>

**Grant Commitments**

From October 1, 2000 until May 31, 2005, AMT received a grant called a "Technisch ontwikkelingskrediet" (TOK) (or technical development loan) from the Dutch government. This TOK grant includes a repayment clause in the event the Company generates revenues from the related project. AMT received total grants of €3,605,000 relating to eligible project costs in the grant period. The grant amount received bears interest of 5.7% per annum and must be repaid in the period January 1, 2008 through December 31, 2017 as a percentage of revenues which are derived from the sale of Glybera. If future royalty payments are not sufficient to repay the grant on or prior to December 3, 2017, or if there are no revenues generated, the remaining balance will be forgiven. Repayment obligations continue to apply if the product is not commercialized or transferred to others. The total amount of the liability at December 31, 2012 was €5,979,000 (2011: €5,657,000), comprising the original total amount of the grant together with accrued interest. The Company has not recorded any liability to repay amounts in respect of this grant within these financial statements. Following the strengthening of the Group's financial position in March 2013 through the extension of the convertible loan, the Company will recognize a charge and a liability within the first quarter of its 2013 financial statements to reflect the repayable amounts that fall due in the change of status of this grant. The Company has commenced repayments of the TOK and associated interest from the commercialization proceeds of Glybera arising from the agreement with Chiesi described in Note 26 below. (Further details are set out in Note 26 below.)

Historically, the Company also received a "Technisch ontwikkelingsproject" (TOP) (or technical development project) grant from the Dutch government amounting to €130,000 on a project that was terminated. If the Company realizes income from the sale of assets developed under that grant, repayment clauses will apply. The Company has not recorded any liability to repay amounts in respect of this grant within these financial statements.

On January 5, 2010, the Company was awarded an investment credit (innovatiekrediet) from the Dutch government (Ministry of Economic Affairs—Agentschap.nl) in respect of the Company's program for Duchenne Muscular Dystrophy. The credit covers 35% of the costs incurred in respect of the program up to a maximum of €4,000,000. The credit includes a repayment clause dependent on the technical success of this program (which is expected to be demonstrated if the product can be successfully commercialized). The credit is interest-bearing at a rate of 11.4% per annum. To date, the Company has received €729,000 under this investment credit, and as of December 31, 2012, the total amount of the liability was €956,000, representing the amount of the original advance together with accrued interest (2011: €858,000). The credit was to be repaid after the funded part of the program was completed in 2013, out

**UNIQUE B.V.****Notes to Consolidated Financial Statements**

of a percentage of revenues derived from the sales resulting from the Company's Duchenne Muscular Dystrophy program. The assets which are financed by means of the investment credit are subject to a right of pledge for the benefit of the Dutch Ministry of Economic Affairs. The project has been terminated following failure to achieve the scientific goals and the Company does not anticipate that any amounts will be realized from this project and consequently there will not be any obligation to repay any of these amounts.

**24. Related-Party Transactions, including Compensation**

Forbion has an interest in the Company in excess of 10%. In addition, Professor Sander van Deventer and Mr Sander Slootweg, who were appointed as members of the Supervisory Board of uniQure on April 5, 2012, are each partners of Forbion. Professor van Deventer also served as a member of the Supervisory Board of AMT for the period from April 28, 2010 to April 5, 2012. Based on the information above, Forbion is a related party of uniQure.

Gilde Healthcare has an interest in the Company in excess of 10%. In addition, Mr Edwin de Graaf, who was appointed as a member of the Supervisory Board of uniQure on April 5, 2012, is a partner of Gilde Healthcare Partners. Based on the information above, Gilde Healthcare is a related party of uniQure.

**Transactions**

The related parties identified above participated in the following transactions during the year ended December 31, 2012:

**Expenses**

The 2009 convertible loan from Forbion accrued interest of 5% during 2012, amounting to €70,000 (2011: €250,000). On April 5, 2012 this loan together with total accrued interest of €320,000 (€70,000 in respect of 2012 and €250,000 in respect of 2011) was converted into 5,320,000 class A ordinary shares, as described in Note 1 above.

The 2012 convertible loan from Forbion, Gilde and other parties (described further in Note 12 above) accrued interest of 8% during 2012, amounting to €4,000.

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## Notes to Consolidated Financial Statements

### Key Management Compensation

The aggregate remuneration of the Supervisory Directors amounted to €255,000 in 2012 (2011: € 174,000) as follows:

	<u>SALARY</u>	<u>BONUS</u>	<u>SHARE-BASED PAYMENTS<sup>(1)</sup></u>	<u>PENSIONS</u>	<u>ADVISOR'S FEE</u>	<u>2012 TOTAL</u>	<u>2011 TOTAL</u>
	(€ in thousands)						
Ferdinand Verdonck	—	—	14	—	29	43	37
Sander van Deventer <sup>(2)</sup>	—	—	—	—	8	8	56
Joseph Feczko	—	—	40	—	29	69	27
Edwin de Graaf <sup>(3)</sup>	—	—	—	—	—	—	—
Francois Meyer	—	—	40	—	29	69	27
Sander Slootweg <sup>(3)</sup>	—	—	—	—	—	—	—
Philippe Van Holle <sup>(4)</sup>	—	—	40	—	26	66	27
Steven Holtzman <sup>(5)</sup>	—	—	—	—	—	—	—
<b>Total</b>	<b>—</b>	<b>—</b>	<b>134</b>	<b>—</b>	<b>121</b>	<b>255</b>	<b>174</b>

(1) The share-based payment reflects the value of equity-settled share options granted during the year, as required by IFRS 2.

(2) Following the combination of uniQure and AMT on April 5, 2012, Professor van Deventer has received no remuneration.

(3) Appointed April 5, 2012; Messrs de Graaf and Slootweg receive no remuneration

(4) Resigned January 1, 2013

(5) Resigned January 3, 2011

The table below sets out a breakdown in the remuneration in 2012 of the members of the Management Board and Senior Management:

<u>DECEMBER 31, 2012</u>	<u>SHORT TERM EMPLOYEE BENEFITS</u>	<u>SHARE- BASED PAYMENTS<sup>(1)</sup></u>	<u>POST- EMPLOYMENT BENEFITS</u>	<u>OTHER LONG TERM BENEFITS</u>	<u>TERMINATION BENEFITS</u>	<u>TOTAL</u>
	(€ in thousands)					
Jörn Aldag	437	359	64	—	—	860
Piers Morgan	258	150	28	—	—	436
<b>Total for Management Directors</b>	<b>695</b>	<b>509</b>	<b>92</b>	<b>—</b>	<b>—</b>	<b>1,296</b>
Senior Management	689	452	41	—	—	1,182
<b>Total</b>	<b>1,384</b>	<b>961</b>	<b>133</b>	<b>—</b>	<b>—</b>	<b>2,478</b>

(1) The share-based payment reflects the value of options granted during the year together with a charge for the period to April 5, 2012 in respect of options granted by AMT.

The total remuneration (excluding share-based payments) paid to or for the benefit of members of the Management Board and Senior Management in 2012 amounted to approximately €1,517,000 (2011: €1,135,000).

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## Notes to Consolidated Financial Statements

The table below sets out a breakdown in the remuneration in 2011 of the members of the Management Board and Senior Management:

<u>DECEMBER 31, 2011</u>	<u>SHORT TERM EMPLOYEE BENEFITS</u>	<u>SHARE- BASED PAYMENTS<sup>(1)</sup></u>	<u>POST- EMPLOYMENT BENEFITS</u>	<u>OTHER LONG TERM BENEFITS</u>	<u>TERMINATION BENEFITS</u>	<u>TOTAL</u>
			(€ in thousands)			
Jörn Aldag	390	267	57	—	—	714
Piers Morgan	227	186	17	—	—	430
<b>Total for Management Directors</b>	<b>617</b>	<b>453</b>	<b>74</b>	<b>—</b>	<b>—</b>	<b>1,144</b>
Senior Management	403	271	41	—	—	715
<b>Total</b>	<b>1,020</b>	<b>724</b>	<b>115</b>	<b>—</b>	<b>—</b>	<b>1,859</b>

<sup>(1)</sup> The share-based payment reflects the value of options granted during the year.

## Shares and Share Options Held by Key Management

### Options

	<u>NUMBER OF OPTIONS AT JANUARY 1, 2012</u>	<u>OPTIONS GRANTED DURING THE YEAR</u>	<u>OPTIONS LAPSED/EXPIRED DURING THE YEAR</u>	<u>NUMBER OF OPTIONS AT DECEMBER 31, 2012</u>
Jörn Aldag	309,400	1,687,825	(309,400)	1,687,825
Piers Morgan	217,600	703,260	(217,600)	703,260
Senior Management	306,000	2,813,040	(306,000)	2,813,040
<b>Total</b>	<b>833,000</b>	<b>5,204,125</b>	<b>(833,000)</b>	<b>5,204,125</b>

### Depository receipts

	<u>NUMBER OF DEPOSITARY RECEIPTS FOR SHARES<sup>(1)</sup></u>
Jörn Aldag	196,945
Piers Morgan	109,712
Senior Management	15,776
<b>Total</b>	<b>322,433</b>

<sup>(1)</sup> These Depository Receipts represent class B ordinary shares.



# UNIQURE B.V.

## Notes to Consolidated Financial Statements

### Receivables and Payables Key Management

(€ in thousands)	DECEMBER 31, 2011	DECEMBER 31, 2012
Receivables from Senior Management	35	26
<b>Total</b>	<b>35</b>	<b>26</b>

These receivables relate to certain wage tax liabilities settled by AMT on behalf of senior management in connection with purchases of AMT depositary receipts in 2007; these amounts are repayable to uniQure on sale of the related depositary receipts or on the respective employee ceasing to be employed by the Company.

### 25. Auditor Services and Fees

Fees paid to the auditors of the Company, PricewaterhouseCoopers Accountants N.V., were as follows:

	2011 (€ in thousands)	2012
Audit fees Annual Report	167	65
Audit fees Half-Year Report	46	—
Tax and HR advisory services	39	5
<b>Total</b>	<b>252</b>	<b>70</b>

### 26. Events after the balance sheet date

Since December 31, 2012 uniQure has entered into certain material agreements, as described in Note 1 above. These agreements do not have a material impact on the results or financial position of uniQure for the period covered by these consolidated financial statements, but are expected to have a material impact in future financial periods.

The grant was received in the period 2001 - 2005 and was treated as income; no liability was recorded historically because repayment was contingent on the commercial success of Glybera.

The completion of the Chiesi agreements on June 30, 2013 (as described in Note 1 above) generated the Company's first revenue in respect of Glybera, in the form of the €2.0m up-front payment received under the commercialization agreement. Under the terms of the TOK described in Note 23 ('Grant commitments'), this triggers repayment obligations to the Dutch Government agency, amounting to 40% of the revenue received, equivalent to €0.8m, which has been paid in September 2013. The repayment obligation is recognized as an expense, including accumulated interest, and as a liability. To the extent that the Company generates further revenue on Glybera it will in future recognize additional expenses and liabilities on an equivalent basis until the full amount of the TOK, together with any accrued interest, is repaid.

Because the relevant event took place after the period covered by these financial statements and does not relate to the position of the Company at December 31, 2012 this event does not result in a change in the financial statements as at December 31, 2012.

**UNIQUE B.V.**

**Notes to Consolidated Financial Statements**

On October 22, 2013, the Company received a demand letter from Extera Partners, a consulting firm based in Boston, US, regarding certain fees alleged to be owed in respect of consulting services provided in connection with a partnering transaction by the Company, under an engagement which expired on December 31, 2012. The total amount claimed by Extera Partners for present and future fees allegedly due as a result of the Company's collaboration agreements with Chiesi, which were entered into in the second quarter of 2013, is said to be in the order to €7,000,000 to €8,000,000; the engagement letter with Extera contained a cap limiting the maximum payment to €5 million. The Company intends to defend the claim vigorously. The Company has reviewed the demand and has determined, on the basis of independent legal advice, that the entire claim is without merit, and consequently it is not expected to have financial consequences for the Company.

No other events occurred after the balance sheet date that would have a material impact on the result or financial position uniQure.

Ordinary Shares

**uniQure**

**uniQure B.V.**

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PRELIMINARY PROSPECTUS

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**Jefferies**

**Leerink Swann**

**Piper Jaffray & Co.**

, 2014

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**PART II****INFORMATION NOT REQUIRED IN PROSPECTUS****Item 6. Indemnification of directors.**

Although Dutch law does not expressly provide for the indemnification of directors, the concept of indemnification of directors of a company for liabilities arising from their actions as members of the management board and supervisory board is, in principle, accepted in the Netherlands. Our articles of association provide for indemnification of the members of the management board and supervisory board by the company to the fullest extent permitted by Dutch law against liabilities, expenses and amounts paid in settlement relating to claims, actions, suits or proceedings to which a director becomes a party as a result of his or her position.

Reference is made to Sections 9 and 10 of the form of Underwriting Agreement filed as Exhibit 1.1 to the registration statement, which sets forth the registrant's and the underwriters' respective agreement to indemnify each other and to provide contribution in circumstances where indemnification is unavailable.

Insofar as indemnification for liabilities arising under the Securities Act may be permitted to directors, officers or persons controlling us pursuant to the foregoing provisions, we have been informed that in the opinion of the Securities and Exchange Commission such indemnification is against public policy as expressed in the Securities Act and is, therefore, unenforceable.

**Item 7. Recent sales of unregistered securities**

Set forth below is information regarding option awards and unrestricted and restricted share issuances made by us since our incorporation in January 2012. Also included is the consideration, if any, received by us for such option awards and shares and information relating to the section of the Securities Act, or rule of the SEC, under which exemption from registration was claimed.

**Option awards**

The table below summarizes all the option awards we have made since our inception pursuant to our 2012 Stock Option Plan. The grant of the option awards and the issuance of ordinary shares upon the exercise of options described in the table below were or will be made pursuant to Regulation S under the Securities Act, or Regulation S, or pursuant to written compensatory plans or arrangements with our employees and directors in reliance on the exemption provided by Rule 701 promulgated under Section 3(b) of the Securities Act, or Rule 701. All recipients either received adequate information about us or had access, through employment or other relationships, to such information.

<u>DATE OF GRANT</u>	<u>NUMBER OF SHARES UNDERLYING SHARE OPTIONS</u>		<u>CURRENT EXERCISE PRICE PER SHARE</u>	
April 5, 2012	6,831,561	€		0.614
June 12, 2012	75,000	€		0.614
December 1, 2012	703,260	€		0.614
December 22, 2012	421,956	€		0.614
January 1, 2013	560,000	€		1.000
March 26, 2013	70,326	€		1.000
June 5/6, 2013	140,000	€		2.020
September 1, 2013	703,260	€		2.020

## Share issuances

In April 2012, we issued 31,101,065 class B ordinary shares to the AMT shareholders as consideration for the business of AMT. This issuance was made outside the United States pursuant to Regulation S or to U.S. persons pursuant to Section 4(2) of the Securities Act. A total of 21,406,311 of these class B ordinary shares were subsequently exchanged into 21,406,311 class A ordinary shares.

In April 2012, we issued 5,320,000 class A ordinary shares to Forbion on the conversion of a convertible loan plus interest amounting in aggregate to €5,320,000. This issuance was made outside the United States pursuant to Regulation S or to U.S. persons pursuant to Section 4(2) of the Securities Act.

In April 2012, we also issued and sold a further 9,771,987 class A ordinary shares to Forbion at a purchase price of €0.614 per share for a total of €6.0 million. This issuance was made outside the United States pursuant to Regulation S or to U.S. persons pursuant to Section 4(2) of the Securities Act.

In April 2012, we also issued and sold a further 1,628,664 class A ordinary shares to Gilde at a purchase price of €0.614 per share for a total of €1.0 million. This issuance was made outside the United States pursuant to Regulation S or to U.S. persons pursuant to Section 4(2) of the Securities Act.

In November 2012, we entered into agreements to raise €0.55 million through the issuance of 899,514 uniQure depository receipts, or DRs, to employees and other persons at a price of €0.614 per uniQure DR. Of these, 445,777 uniQure DRs, representing approximately €0.27 million, were issued prior to December 31, 2012, and the remaining 453,737 uniQure DRs, representing approximately €0.28 million, were issued after December 31, 2012. These issuances were made outside the United States pursuant to Regulation S or to U.S. persons pursuant to Section 4(2) of the Securities Act.

In December 2012, January 2013 and March 2013, we sold convertible promissory notes in the aggregate principal amount of €13.5 million in a private placement to certain of our existing investors. In July 2013, these convertible notes converted into 6,681,678 of our class A ordinary shares. This issuance was made outside the United States pursuant to Regulation S.

In July 2013, we issued and sold to Chiesi Farmaceutici S.p.A. 5,546,070 of our class C ordinary shares at a purchase price of €2.52 per share for a total of €14.0 million. This issuance was made outside the United States pursuant to Regulation S.

In November 2013, we also issued and sold to certain individuals a total of 25,592 uniQure DRs at a price of €0.614 per uniQure DR for a total of €16,000, pursuant to the exercise of certain options granted on June 12, 2012. This issuance was made outside the United States pursuant to Regulation S or to U.S. persons pursuant to Section 4(2) of the Securities Act.

## Item 8. Exhibits and financial statement schedules

- (a) The Exhibit Index is incorporated herein by reference.
- (b) Financial Statement Schedules.

Schedules have been omitted because the information required to be set forth therein is not applicable or is shown in the consolidated financial statements or the notes thereto.

## Item 9. Undertakings

- (a) The undersigned registrant hereby undertakes to provide to the underwriters at the closing specified in the underwriting agreement certificates in such denominations and registered in such names as required by the underwriters to permit prompt delivery to each purchaser.

- (b) Insofar as indemnification for liabilities arising under the Securities Act may be permitted to directors, officers and controlling persons of the registrant pursuant to the foregoing provisions, or otherwise, the registrant has been advised that in the opinion of the SEC such indemnification is against public policy as expressed in the Act and is, therefore, unenforceable. In the event that a claim for indemnification against such liabilities (other than the payment by the registrant of expenses incurred or paid by a director, officer or controlling person of the registrant in the successful defense of any action, suit or proceeding) is asserted by such director, officer or controlling person in connection with the securities being registered, the registrant will, unless in the opinion of its counsel the matter has been settled by controlling precedent, submit to a court of appropriate jurisdiction the question whether such indemnification by it is against public policy as expressed in the Act and will be governed by the final adjudication of such issue.
- (c) The undersigned registrant hereby undertakes that:
  - (1) For purposes of determining any liability under the Securities Act, the information omitted from the form of prospectus filed as part of this registration statement in reliance upon Rule 430A and contained in a form of prospectus filed by the registrant pursuant to Rule 424(b)(1) or (4) or 497(h) under the Securities Act shall be deemed to be part of this registration statement as of the time it was declared effective.
  - (2) For the purpose of determining any liability under the Securities Act, each post-effective amendment that contains a form of prospectus shall be deemed to be a new registration statement relating to the securities offered therein, and the offering of such securities at that time shall be deemed to be the initial bona fide offering thereof.

## SIGNATURES

Pursuant to the requirements of the Securities Act of 1933, the registrant certifies that it has reasonable grounds to believe that it meets all of the requirements for filing on Form F-1 and has duly caused this registration statement to be signed on its behalf by the undersigned, thereunto duly authorized, on January 2, 2014.

### UNIQUE B.V.

By: /s/ JÖRN ALDAG

Name: **Jörn Aldag**

Title: **Chief Executive Officer**

## POWER OF ATTORNEY

Each person whose signature appears below constitutes and appoints Jörn Aldag and Piers Morgan, and each of them, as attorney-in-fact with full power of substitution, for him or her in any and all capacities, to do any and all acts and all things and to execute any and all instruments which said attorney and agent may deem necessary or desirable to enable the registrant to comply with the Securities Act, and any rules, regulations and requirements of the Securities and Exchange Commission thereunder, in connection with the registration under the Securities Act of ordinary shares of the registrant (the "Shares"), including, without limitation, the power and authority to sign the name of each of the undersigned in the capacities indicated below to the Registration Statement on Form F-1 (the "Registration Statement") to be filed with the Securities and Exchange Commission with respect to such Shares, to any and all amendments or supplements to such Registration Statement, whether such amendments or supplements are filed before or after the effective date of such Registration Statement, to any related Registration Statement filed pursuant to Rule 462(b) under the Securities Act, and to any and all instruments or documents filed as part of or in connection with such Registration Statement or any and all amendments thereto, whether such amendments are filed before or after the effective date of such Registration Statement; and each of the undersigned hereby ratifies and confirms all that such attorney and agent shall do or cause to be done by virtue hereof.

Pursuant to the requirements of the Securities Act of 1933, this registration statement has been signed by the following persons in the capacities and on the dates indicated.

<u>SIGNATURES</u>	<u>TITLE</u>	<u>DATE</u>
<u>/s/ JÖRN ALDAG</u> <b>Jörn Aldag</b>	Chief Executive Officer (Principal Executive Officer)	December 30, 2013
<u>/s/ PIERS MORGAN</u> <b>Piers Morgan</b>	Chief Financial Officer (Principal Financial and Accounting Officer)	January 2, 2014
<u>/s/ FERDINAND VERDONCK</u> <b>Ferdinand Verdonck</b>	Chairman	December 26, 2013

<u>SIGNATURES</u>	<u>TITLE</u>	<u>DATE</u>
<u>/s/ SANDER SLOOTWEG</u> <b>Sander Slootweg</b>	Non-Executive Director	January 2, 2014
<u>/s/ SANDER VAN DEVENTER</u> <b>Sander van Deventer</b>	Non-Executive Director	December 31, 2013
<u>/s/ JOSEPH M. FECZKO</u> <b>Joseph M. Feczko</b>	Non-Executive Director	January 2, 2014
<u>/s/ FRANÇOIS MEYER</u> <b>François Meyer</b>	Non-Executive Director	January 2, 2014
<u>/s/ PAULA SOTEROPOULOS</u> <b>Paula Soteropoulos</b>	Non-Executive Director	January 2, 2014
<b>UNIQUE INC.</b> Authorized Representative in the United States		
<b>By:</b> <u>/s/ PHILIP ASTLEY-SPARKE</u>		
Name: <b>Philip Astley-Sparke</b> Title: <b>President, US Operations</b>		January 2, 2014



## EXHIBIT INDEX

Exhibit No.	Description
1.1*	Form of Underwriting Agreement
3.1	Articles of Association of the Registrant as in effect prior to this offering
3.2*	Amended Articles of Association of the Registrant to be effective upon the closing of this offering
4.2	Class A Shareholders Agreement, dated as of April 19, 2012, by and among Cooperatieve AAC LS U.A., Forbion Co-Investment Cooperatief U.A., Forbion Co-Investment II Cooperatief U.A., Cooperatieve Gilde Healthcare II U.A. and the Registrant
4.3	Accession Agreement to the Class A Shareholders Agreement, dated as of May 25, 2012, by and among the Registrant and the Parties listed therein
4.4	Class B Shareholders Agreement, dated as of April 19, 2012, by and among Cooperatieve AAC LS U.A., Forbion Co-Investment Cooperatief U.A., Forbion Co-Investment II Cooperatief U.A., Cooperatieve Gilde Healthcare II U.A., Stichting Administratiekantoor uniQuire B.V. and the Registrant
4.5	Accession Agreement to the Class B Shareholders Agreement, dated as of May 25, 2012, by and among the Registrant and the Parties listed therein
4.6	Class C Shareholders Agreement, dated as of July 8, 2013, by and among the Registrant and the Parties listed therein.
5.1	Form of Opinion of Rutgers Posch Visée Endedijk N.V.
8.1*	Form of Tax Opinion of WilmerHale LLP
8.2	Form of Tax Opinion of Liem & Partners N.V.
10.1†	Patent License Agreement (L-107-2007), effective as of May 2, 2007, by and between the Registrant and the National Institutes of Health, as amended on December 31, 2009, May 31, 2013 and November 11, 2013
10.2†	Patent License Agreement (L-116-2011), effective as of August 10, 2011, by and between the Registrant and National Institutes of Health, as amended on May 31, 2013 and November 11, 2013
10.3†	License Agreement, effective as of March 22, 2007, by and between the Registrant and Protein Sciences Corporation, as amended on June 13, 2012
10.4†	Agreement, dated June 16, 2006, by and among the Registrant, Academisch Medisch Centrum and Beheersmaatschappij Dienstverlening En Deelneming Azua
10.5†*	Sublicense and Research Agreement, effective June 18, 2001, by and between the Registrant and Xenon Genetics Inc., as amended
10.6†	License Agreement, effective as of December 20, 2006, between the Registrant and Aventis Pharma S.A., as amended on June 28, 2013
10.7†	Non-Exclusive License Agreement, effective as of September 3, 2010, by and between the Registrant and Asklepios Biopharmaceutical, Inc.
10.8†	License Agreement, dated February 8, 2008, by and between the Registrant and Salk Institute for Biological Studies
10.9†	License Agreement, dated December 5, 2006, by and between the Registrant and AmpliPhi Biosciences, Inc., as amended on June 28, 2013
10.10†	Exclusive License Agreement, effective as of July 7, 2008, by and between the Registrant and St. Jude Children's Research Hospital, Inc., as amended on July 12, 2012
10.11†	Co-Development and License Agreement, entered into as of April 29, 2013, by and between the Registrant and Chiesi Farmaceutici S.p.A.

Exhibit No.	Description
10.12†	Commercialization Agreement, entered into as of April 29, 2013, by and between the Registrant and Chiesi Farmaceutici S.p.A.
10.13†	License Agreement, dated as of May 21, 2010, by and among the Registrant, Fundacion para la Investigacion Medica Aplicada, Proyecto de Biomedicina CIMA S.L. and Digna Biotech, S.L.
10.14†	Development and Manufacturing Agreement, effective as of January 7, 2011, by and between the Registrant and Institut Pasteur, as amended on January 7, 2011
10.15†	License Agreement, effective as of November 30, 2010, by and between the Registrant and Amgen Inc.
10.16†	Data License Agreement, effective June 12, 2012, by and between the Registrant and The Regents of the University of California, acting through its Office of Technology management, University of California, San Francisco
10.17	Loan and Security Agreement, dated as of June 13, 2013, by and among the Registrant, uniQure IP B.V., the Registrant's subsidiaries listed therein, and Hercules Technology Growth Capital, Inc.
10.18	Warrant Agreement, dated as of September 20, 2013, by and among the Registrant, uniQure Biopharma B.V. and Hercules Technology Growth Capital, Inc.
10.19	Subscription Agreement, dated as of April 29, 2013, by and among Chiesi Farmaceutici S.p.A and the Registrant
10.20+	2012 Option Plan
10.21+	Form of Grant letter under the 2012 Option Plan
10.22+*	2013 Share Incentive Plan
10.23+*	Form of Incentive Share Option Agreement under the 2013 Share Incentive Plan
10.24+*	Form of Non-Qualified Share Option Agreement under the 2013 Share Incentive Plan
10.25*	Form of Appointment Letter for Supervisory Directors
10.26*	Lease relating to Meibergdreef 61, dated as of October 19, 2005, by and among Beheermaatschappij Dienstverlening en Deelneming AZUA B.V. and Amsterdam Molecular Therapeutics B.V.
10.27*	Lease relating to Meibergdreef 57, dated as of October 1, 2005, by and among Beheermaatschappij Dienstverlening en Deelneming AZUA B.V. and Amsterdam Molecular Therapeutics B.V.
10.28	Lease relating to 113 Hartwell Avenue, Lexington, Massachusetts, dated as of July 24, 2013, by and between the Registrant and King113 Hartwell LLC
10.29	Business Acquisition Agreement, dated as of February 16, 2012, by and among Amsterdam Molecular Therapeutics (AMT) Holding N.V., the Registrant and the other Parties listed therein
10.30	Agreement for Transfer of Certain Assets and Liabilities of Amsterdam Molecular Therapeutics (AMT) Holding N.V., dated as of February 16, 2012, by and among Amsterdam Molecular Therapeutics (AMT) Holding B.V., Amsterdam Molecular Therapeutics (AMT) Holding IP B.V. and Amsterdam Molecular Therapeutics (AMT) Holding N.V.
10.31	Deed of Assignment of Certain Assets and Liabilities of Amsterdam Molecular Therapeutics (AMT) Holding N.V., dated as of April 5, 2012, by and among Amsterdam Molecular Therapeutics (AMT) Holding B.V., Amsterdam Molecular Therapeutics (AMT) Holding IP B.V. and Amsterdam Molecular Therapeutics (AMT) Holding N.V.
21.1	Subsidiaries of the Registrant

<b>Exhibit No.</b>	<b>Description</b>
23.1	Consent of PricewaterhouseCoopers Accountants N.V.
23.2	Form of Consent of Rutgers Posch Visée Endedijk N.V. (included in Exhibit 5.1)
23.3*	Form of Consent of WilmerHale LLP (included in Exhibit 8.1)
23.4	Form of Consent of Liem & Partners N.V. (included in Exhibit 8.2)
24.1	Powers of Attorney (included on signature page)
99.1	Registrant's Application for Waiver of Requirements of Form 20-F, Item 8.A.4

\* To be filed by amendment

† Confidential treatment requested as to certain portions, which portions have been omitted and filed separately with the Securities and Exchange Commission

+ Management contract or compensatory plan or arrangement



## ENGLISH ARTICLES OF ASSOCIATION OF UNIQUE B.V. IN FORCE AS OF

24 JULY 2013

following a deed of amendment executed before a substitute of Cornelia Holdinga, civil law notary in Amsterdam.

## NOTE ABOUT TRANSLATION:

This document is an English translation of a document prepared in Dutch. In preparing this document, an attempt has been made to translate as literally as possible without jeopardizing the overall continuity of the text. Inevitably, however, differences may occur in translation and if they do, the Dutch text will govern by law.

In this translation, Dutch legal concepts are expressed in English terms and not in their original Dutch terms. The concepts concerned may not be identical to concepts described by the English terms as such terms may be understood under the laws of other jurisdictions.

## ARTICLES OF ASSOCIATION UNIQUE B.V.

## 1. DEFINITIONS

1.1. In these articles capitalised terms shall have the meaning ascribed to these terms as follows:

- (a) **‘Shareholders’** shall mean the holders of Class A Shares, the holders of Class B, the holders of Class C Shares, unless the contrary has been stated explicitly or appears from the context;
- (b) **‘Class A Shares’** shall mean registered class A Shares in the capital of the Company, each with a nominal value of one euro cent (EUR 0.01);
- (c) **‘Class B Shares’** shall mean registered convertible class B Shares in the capital of the Company, each with a nominal value of one euro cent (EUR 0.01);
- (d) **‘Class C Shares’** shall mean registered class C Shares in the capital of the Company, each with a nominal value of one euro cent (EUR 0.01);
- (e) **‘Shares’** shall mean the Class A Shares, the Class B Shares and the Class C Shares, unless the contrary has been stated explicitly or appears from the context;
- (f) **‘Absolute Shareholder Class A Majority’** shall mean an affirmative vote of the Class A Shareholders representing at least fifty-one per cent (51%) of the issued and outstanding Shares Class A;
- (g) **‘Absolute Shareholder Class B Majority’** shall mean an affirmative vote of the Class B Shareholders representing at least fifty-one per cent (51%) of the issued and outstanding Shares Class B;

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- (h) **‘Absolute Shareholder Class C Majority’** shall mean an affirmative vote of the Class C Shareholders representing at least fifty-one per cent (51%) of the issued and outstanding Shares Class C;
  - (i) **‘Absolute Shareholder Majority’** shall mean an affirmative vote of the Shareholders representing at least fifty-one per cent (51%) of the issued and outstanding Shares;
  - (j) **‘Business’** shall mean all activities of the Group;
  - (k) **‘General Meeting’** shall mean the body corporate formed by the Shareholders or the meeting of said body, as the case may be;
  - (l) **‘GM Resolution’** shall mean a resolution of the General Meeting, adopted in a meeting or outside a meeting, as the case may be;
  - (m) **‘Management Board’** shall mean the Management Board of the Company appointed in accordance with article 13.1;
  - (n) **‘Management Board Member’** shall mean a member of the Management Board;
  - (o) **‘Supervisory Board Members A’** shall mean the members of the Supervisory Board appointed in accordance with article 17.3;
  - (p) **‘Supervisory Board Members B’** shall mean the members of the Supervisory Board appointed in accordance with article 17.4;
  - (q) **‘Supervisory Board Member’** shall mean a member of the Supervisory Board;
  - (r) **‘Qualified Class A Majority’** shall mean the affirmative vote of the holders of Class A Shares representing at least two third of the issued and outstanding Class A Shares;
  - (s) **‘Qualified Shareholder Majority’** shall mean the affirmative vote of the Shareholders representing at least two third of the issued and outstanding Shares;
  - (t) **‘Group’** shall mean the Company and its subsidiaries;

- (u) **‘Group Company’** shall mean a member of the Group;
- (v) **‘Supervisory Board’** shall mean the supervisory board of the Company appointed in accordance with article 17;
- (w) **‘Company’** shall mean the private company with limited liability (besloten vennootschap met beperkte aansprakelijkheid) uniQure B.V., having its seat in Amsterdam and registered with the trade register under number 54385229;
- (x) **‘Class A Meeting’** shall mean the body corporate formed by the holders of Class A Shares or the meeting of said body, as the case may be;

- (y) **‘Class B Meeting’** shall mean the body corporate formed by the holders of Class B Shares or the meeting of said body, as the case may be.
- (z) **‘Class C Meeting’** shall mean the body corporate formed by the holders of Class C Shares or the meeting of said body, as the case may be.

1.2. The definitions described in article 1.1 shall apply both to the singular and the plural of the concepts defined.

1.3. All references to ‘articles’ shall be deemed to be a reference to articles in these articles of association, unless it is indicated otherwise.

## 2. NAME AND SEAT

2.1. The name of the Company is: uniQure B.V.

2.2. The Company has its seat in Amsterdam.

## 3. OBJECTS

The objects of the Company are:

- to research, develop, produce and commercialise products, services and technology in the (bio-)pharmaceutical sphere;
- to participate in, to finance, to collaborate with, to conduct the management of companies and other enterprises and to provide advice and other services;
- to acquire, use and/or assign industrial and intellectual property rights and real property;
- to invest funds;
- to provide security for the debts of legal persons or of other companies with which the Company is affiliated in a group or for the debts of third parties;
- to undertake all that is connected to the foregoing or in furtherance thereof,

all in the widest sense of the words.

## 4. CAPITAL AND SHARES

4.1. The company’s authorised capital amounts to two million euro (EUR 2.000.000) and is divided into one hundred and seventy-one million four hundred and six thousand three hundred and eleven (171,406,311) Class A shares, eighteen million five hundred and ninety-three thousand six hundred and eighty-nine (18,593,689) convertible Class B shares, ten million (10,000,000) Class C Shares, each with a nominal value of one euro cent (EUR 0.01).

4.2. The Shares shall be registered and numbered per class consecutively from 1 upwards with denomination of the letter corresponding with the relevant class of Shares. The Company shall not issue share certificates.

4.3. Pursuant to the provisions of article 23 Class B Shares may be converted into Class A Shares.

As a result of such conversion the number of Class A Shares included in the authorised share capital of the Company shall be increased and the number of Class B Shares included in the authorised share capital of the Company shall be decreased with the number of Class B Shares so converted.

Class A Shares may not be converted into Class B Shares nor into Class C Shares. Class C Shares are not convertible into either A or B.

## 5. THE ISSUE OF SHARES

5.1. Subject to the prior approval of the Class A Meeting, the issue of Shares may only be effected through a resolution of the General Meeting, which shall also set out the price and the other terms and conditions of issue, if any. Furthermore, the issue of Shares requires a notarial deed to that effect, executed before a civil-law notary, officiating in the Netherlands.

The issue price may not be below par. Upon subscription for Shares the nominal amount must be paid up.

5.2. Subject to the prior approval of the Class A Meeting, the General Meeting may delegate the powers set forth in article 5.1 and in article 5.3, to another corporate body of the Company and may also revoke any such delegation.

5.3. Each Shareholder shall, with respect to any issue of Shares, have a pre-emptive right in proportion to the aggregate amount of Shares held by him.

This pre-emptive right is non-transferable.

Subject to the prior approval of the Class A Meeting, the General Meeting may limit or exclude the pre-emptive right with respect to a specific issue.

5.4. The provisions of the above paragraphs of the present article shall apply accordingly to the granting of a right to subscribe for Shares (including but not limited to option rights and/or warrants), but shall not apply to the issue of Shares to someone who exercises a previously acquired subscription right.

## **6. OWN SHARES**

6.1. The Company may acquire fully paid-up Shares in its capital or depositary receipts issued for such Shares without paying any consideration with due observance of article 2:207 of the Dutch Civil Code and only pursuant to a resolution of the General Meeting adopted with the prior approval of the Class A Meeting.

6.2. The provisions of article 5 shall apply accordingly to the disposal of Shares that the Company holds in its own capital, except that such disposal may be below par.

The provisions of article 11 shall not apply to the disposal of Shares that the Company holds in its own capital. The approval for the transfer within the meaning of article 2:195 paragraph 4 Dutch Civil Code is considered to be

granted in the resolution to dispose of Shares that the Company holds in its own capital.

6.3. The term 'Shares' as used in this article shall include depositary receipts issued for Shares.

## **7. REDUCTION OF CAPITAL**

7.1. The General Meeting may resolve to reduce the issued capital by cancellation of Shares or by amending the articles to provide for a reduction of the par value of the Shares, provided that the resolution to amend the articles of association in order to reduce the par value of the Shares Class B can only be adopted with the prior approval of the Class B and provided that the resolution to amend the articles of association in order to reduce the par value of the Shares Class C can only be adopted with the prior approval of the Class C Meeting.

The Shares referred to in any such resolution must be specified therein and provisions for the implementation of such resolution must be made therein.

The paid in capital may not fall below the minimum capital required by law at the time of the resolution.

7.2. A resolution to cancel Shares may relate only to:

- Shares held by the Company itself or with respect to Shares of which it holds the depositary receipts;
- all Shares of a class with the consent of all Shareholders of such class; or
- all Shares of a class with repayment.

7.3. Any reduction of the par value of Shares without redemption and without a release from the obligation to pay up, must be made in proportion to all Shares of that class. This proportional requirement may be waived by agreement of all Shareholders affected.

7.4. The notice convening a meeting at which a resolution referred to in this article is to be adopted shall state the purpose of the reduction of capital and the manner of its implementation.

7.5. Any GM Resolution referred to in this article can only be adopted after having obtained the prior approval of the Class A Meeting.

## **8. REGISTER OF SHAREHOLDERS**

8.1. The Management Board shall keep a register in which the names and addresses of all Shareholders per class are recorded, stating the number and class of Shares held by them, and numbers allocated to such Shares, the date on which the Shares were acquired, the date of the acknowledgement of the transfer or the date on which the transfer has been served upon the Company, as well as the amount paid-up on each Share.

The register shall also record the names and addresses of the usufructuaries of Shares, showing the date on which they acquired the right on the Shares, to

which Shares their rights are attached, the date of the acknowledgement of the acquisition of the right of usufruct or the date on which such acquisition has been served on the Company, and which rights they have.

8.2. The register shall be updated regularly.

8.3. Each Shareholder and usufructuary shall ensure that the Company is informed of his address.

8.4. If a Shareholder or a usufructuary also disclosed an electronic address to the Company for the purposes of entering this electronic address, together with the other data mentioned in article 8.1, into the register, such disclosure is deemed to entail the consent to receive all notifications and announcements as well as convening notices for General Meetings electronically.

The provision of the previous sentence regarding the notice for General Meetings shall only apply to the extent the usufructuary is entitled to attend the meeting.

A message sent electronically needs to be legible and reproducible.

## **9. USUFRUCT AND PLEDGE ON SHARES**

9.1. A usufruct may be imposed on Shares. Shares may not be pledged.

9.2. Only the Shareholder shall have the voting rights with respect to Shares subject to a usufruct.

9.3. Contrary to the provisions of article 9.2, the usufructuary shall have the voting rights if so provided at the time of granting the usufruct, provided that both such provision and, in case of a transfer of the usufruct, the assignment of the voting rights is approved by the General Meeting.

## **10. NO COOPERATION DEPOSITARY RECEIPTS**

Depository receipts issued for shares, if any, will not carry any meeting rights as defined in article 2: 227 Dutch Civil Code.

## **11. RESTRICTION ON THE TRANSFER OF SHARES**

11.1. Shares may be transferred only after the approval for the proposed transfer has been granted by the Class A Meeting.

A restriction on the transfer of Shares as mentioned in this article shall not apply if the Shareholder must transfer his Share to a previous holder pursuant to the law.

The provisions of the present article shall apply accordingly to the assignment of a right to subscribe for Shares (including but not limited to option rights and/or warrants).

11.2. The approval shall be applied for by way of a letter addressed to the Company, setting out the number of Shares for which a decision is sought and the name of the person to whom the applicant wishes to make the transfer.

11.3. The request is deemed to have been approved if:

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- the applicant has not been informed of a decision within three months of the receipt of the request; or

- together with a rejection of the application, the applicant is not notified by the Class A Meeting of a prospective purchaser(s) willing and able to purchase all of the Shares for cash.

11.4. In case the approval is granted or is deemed to have been granted, the applicant is free to transfer his Shares for a period of three months after the receipt of a notice that the approval has been granted or is deemed to have been granted, or after the period referred to in article 11.3 has expired.

11.5. The Company itself may be a prospective purchaser only after obtaining the consent of the applicant.

11.6. The price for which the prospective purchaser(s) appointed by the Class A Meeting may purchase the Shares, shall be determined by mutual agreement between the applicant and the appointed prospective purchaser(s). However, upon request of the applicant the price shall be determined by an independent expert to be appointed by the sub-district court of the Court (kantonrechter van de Rechtbank) within which district the Company has its seat.

11.7. The remuneration to be paid to the expert shall be borne by the applicant and the appointed prospective purchaser(s). Unless otherwise agreed by the applicant and the prospective purchaser(s), the total amount to be paid shall be born by the applicant for one-half and the joint prospective purchaser(s) for the other half, in proportion to the number of Shares designated to each prospective purchaser.

11.8. The applicant may withdraw his request within one month of having been notified of the price as determined by the expert. In such case the remuneration referred to in article 11.6, if any, shall be entirely borne by the applicant.

## **12. TRANSFER OF SHARES AND LIMITED RIGHTS**

The transfer of a Share or the creation or assignment of a limited right on a Share requires a notarial deed to that effect, executed before a civil-law notary, officiating in the Netherlands.

## **13. MANAGEMENT BOARD**

13.1. The Company will be managed by a Management Board consisting of two or more members, to be determined by the General Meeting.

13.2. Management Board Members shall be appointed by the General Meeting from a binding nomination drawn up by the Class A Meeting.

The binding nomination must be drawn up within two months after the vacancy has occurred.

If the Class A Meeting should fail to exercise its right to draw up a binding nomination or should fail to do so in a timely manner, the General Meeting shall be free in its choice.

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- 13.3. Management Board Members may at any time be suspended or dismissed by the General Meeting.
- 13.4. Management Board Members may be suspended by the Supervisory Board at any time.
- 13.5. A suspension may last no longer than three months in total, even after having been extended one or more times, unless a resolution for dismissal is adopted, in which case this term runs until the end of the employment contract.
- 13.6. The determination or variation of the remuneration and other terms and conditions under which each individual Management Board Member is appointed shall be determined by the General Meeting with the prior approval of the Class A Meeting.

#### **14. MANAGEMENT DUTY. APPROVALS**

- 14.1. With due observance of the limitations set out by the present articles of association, the Management Board is charged with the management of the Company.
- 14.2. The Management Board is authorised to appoint one or more persons with the authority to represent the Company and, by granting a power of attorney, conferring such titles and powers as the Management Board shall determine.
- 14.3. The Management Board shall adopt resolutions by an absolute majority of the votes cast. Blank votes shall be considered null and void.
- Each Management Board Member may cast one vote, notwithstanding the applicable statutory provisions.
- 14.4. In addition to the relevant provisions of these articles of association, the Management Board may adopt internal rules with respect to the holding of meetings and its decision-making process. These rules may include an internal allocation of duties among the Management Board.
- 14.5. A Management Board Member may be represented at Management Board meetings only by another Management Board Member, and only for a specific meeting.
- 14.6. The Management Board may also adopt resolutions without holding a meeting, provided that all Management Board Members have been consulted and none of them have objected to resolutions being adopted in this manner.
- 14.7. With due observance of the provisions of these articles, the Management Board resolutions relating to any of the following matters shall be subject to the approval of the Supervisory Board:
- (a) any amendment to, or any proposal to amend, the articles of association of any Group Company;
  - (b) the instigation or the settlement of any material litigation or arbitration or mediation proceedings by a Group Company, for the purposes of which material shall mean an interest or claim that is of strategic

importance to the Group or has a monetary value of at least one hundred thousand euros (EUR 100,000);

- (c) any proposals to the General Meeting to materially change the emoluments of members of the Management Board, including bonuses and option schemes;
  - (d) the removal or appointment of the auditors of any Group Company, other than the reappointment of existing auditors;
  - (e) remuneration of the auditors of the Company;
  - (f) approval of any change in accounting policies of any Group Company;
  - (g) alteration to the financial year end of any Group Company;
  - (h) non-project related capital expenditures exceeding in one transaction or event or series of related transactions or events, an amount of fifty thousand euros (EUR 50,000) but less than one hundred thousand euros (EUR 100,000), which are not included in an approved business plan or budget.
- 14.8. With due observance of the provisions of these articles, the Management Board resolutions relating to any of the following matters shall be subject to the approval of the Class A Meeting:
- (a) unless specified in an approved business plan of the Company, entering into or materially changing borrowing and lending arrangements (including issuance of debt instruments) by any Group Company, exceeding two hundred and fifty thousand euros (EUR 250,000);
  - (b) unless specified in an approved business plan of the Company, establishing and/or closing any material branch, establishment, agency or business of any Group Company;
  - (c) unless specified in an approved business plan of the Company, entering into any material joint venture, partnership or profit sharing arrangement or licensing agreement by any Group Company;
  - (d) unless specified in an approved business plan of the Company, the expansion or development of the Group or any of its business other than through a Group Company;
  - (e) adoption of or amendment to the business plan and budget;

- (f) creation or release of any security or (save in the ordinary course of trading and consistent with past practice) granting of guarantees by any Group Company, exceeding an amount of two hundred and fifty thousand euros (EUR 250,000);
- (g) unless specified in an approved business plan of the Company, any material acquisitions or disposals by any Group Company;
- (h) the appointment or removal of any member of the supervisory board or managing director of a Group Company (other than the Company);

- (i) non-project related capital expenditures exceeding in one transaction or event or series of related transactions or events, in an amount of one hundred thousand euros (EUR 100,000) or more, which are not included in an approved business plan or budget;
- (j) establishment and material amendment of any management incentive scheme of any Group Company (other than the Company).

14.9. With due observance of the provisions of these articles, the Management Board resolutions relating to any of the following matters shall be subject to the approval of the Class A Meeting, which resolution can only be adopted with a Qualified Class A Majority:

- (a) any change in a Group Company's (other than the Company's) share capital;
- (b) unless specified in an approved business plan of the Company, any material change of the nature or the name of the business of the Group;
- (c) entry into, termination or variation of any contract or arrangement by a Group Company with a Class A Shareholder, other than financing arrangements;
- (d) any distribution from reserves (other than wholly intra-group) by any Group Company;
- (e) transactions by a Group Company outside of its ordinary course;
- (f) taking steps to commence insolvency or winding-up proceedings of a Group Company (including the application for suspension of payment of debts by a Group Company and the filing for bankruptcy of the Company).

14.10. A resolution of the Management Board to exercise the Company's voting rights in a Group Company, is also subject to the prior approval of the Supervisory Board and/or the Class A Meeting, as the case may be, if it concerns a resolution of the General Meeting of such company which relates to any of the matters included in this article.

14.11. Failure to obtain the approval as referred to in this article shall not affect the authority of the Management Board or the Management Board Members to represent the Company.

14.12. The Management Board is not authorised to engage in the legal transactions (rechtshandelingen) set forth in article 2:204 paragraph 1 Dutch Civil Code without obtaining the prior approval of the General Meeting.

## 15. ABSENCE AND PREVENTION

In the event that one or more Management Board Members are absent or prevented from acting, the remaining Management Board Members or the sole remaining Management Board Member shall be entrusted with the management of the Company.

In the event that all Management Board Members are absent or prevented from acting, two persons to be appointed for that purpose by the Supervisory Board, whether or not from among its members, shall be temporarily entrusted with the management of the Company.

Only a Supervisory Board Member as referred to in article 17.3 may be appointed in accordance with the previous sentence.

## 16. REPRESENTATION

16.1. The Company shall be represented by the Management Board.

Except for the Management Board, the authority to represent the Company is only vested in two Management Board Members acting jointly.

16.2. In all events in which the Company has a direct or indirect conflict of interest with a Management Board Member in his private capacity, the Management Board resolution regarding that relevant legal act requires the prior approval of the Supervisory Board.

Failure to obtain the approval defined in the previous sentence shall not affect the authority of the Management Board or the Management Board Member(s) to represent the Company.

## 17. SUPERVISORY BOARD

17.1. The Company shall have a Supervisory Board consisting of up to seven (7) Supervisory Board Members who shall have the title Supervisory Board Member A or Supervisory Board Member B, provided that at all times there shall be at least one Supervisory Board Member B more than there are Supervisory Board Members A.

With due observance of the previous sentence, the exact number of Supervisory Board Members shall be determined by the Class A Meeting.

Only natural persons can be appointed to the Supervisory Board.

17.2. The duties of the Supervisory Board shall be the supervision of the conduct of management by the Management Board and of the general course of affairs of the Company and of any affiliated enterprise.

The Supervisory Board shall assist the Management Board by rendering advice.

In performing their duties, the Supervisory Board Members shall be guided by the interests of the Company and of any enterprise affiliated therewith.

The Management Board shall provide the Supervisory Board with the information necessary for the performance of its duties, in a timely manner.

17.3. Supervisory Board Members A shall be appointed by the General Meeting from a binding nomination drawn up by the Class A Meeting.

Prior to the making of a nomination, the Class B Meeting shall be consulted on the identity and the qualifications of the persons to be potentially nominated.

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Any substantial objections against potential nominees shall be taken into account when making the actual nomination.

17.4. Supervisory Board Members B shall be appointed by the General Meeting.

17.5. The determination or variation of the remuneration and other terms and conditions under which each individual member of the Supervisory Board is appointed shall be determined by the General Meeting with the prior approval of the Class A Meeting.

17.6. A Supervisory Board Member shall resign at the close of the General Meeting at which the annual accounts are considered, in the year in which four years have elapsed since his latest appointment.

Resigning members of the Supervisory Board shall be immediately eligible for re-election.

Members of the Supervisory Board may be suspended or dismissed by the General Meeting at any time.

A suspension may last no longer than three months in total, even after having been extended one or more times.

17.7. The Supervisory Board shall at any time have access to all buildings and premises in use by the Company, and shall be entitled to inspect all of the Company's books and records and to examine all of the Company's assets.

The Supervisory Board may delegate this authority to one or more of its members, or to an expert.

17.8. The Supervisory Board shall appoint a chairman from among its Supervisory Board Members A or by the Supervisory Board Member B nominated by the Class A Shares Meeting.

17.9. The Supervisory Board shall have at least two standing committees, being the audit committee and the remuneration and appointment committee to be appointed by the Supervisory Board from its own members.

17.10. The General Meeting may designate a Supervisory Board Member as delegated member of the Supervisory Board who shall be particularly responsible for maintaining regular contact with the Management Board on the state of affairs in the Company.

17.11. The Supervisory Board shall hold at least six (6) meetings a year. Additional meetings may be held at the discretion of the chairman of the Supervisory Board or as often as one or more Supervisory Board Members shall desire or as often as the Management Board shall request.

17.12. The Supervisory Board shall adopt resolutions by an absolute majority of the total number of votes cast. Blank votes shall be considered null and void. Each member of the Supervisory Board shall be entitled to cast one vote. In case the votes tie, the chairman of the Supervisory Board shall have a casting vote.

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17.13. A Supervisory Board Member may be represented at a meeting of the Supervisory Board only by another Supervisory Board Member and only for a specific meeting.

17.14. Meetings of the Supervisory Board may also be held by telephone or video conference.

17.15. The Supervisory Board may also adopt valid resolutions without convening a meeting, provided that all of its members have been consulted and that none has objected to adopting resolutions in this manner.

17.16. If it is necessary to provide the Shareholders or the Management Board with evidence of a resolution adopted by the Supervisory Board, the signature of either the chairman of the Supervisory Board or of the delegated member of the Supervisory Board, as referred to in article 17.10 shall suffice.

17.17. In the event that one or more Supervisory Board Members are absent or prevented from acting, the remaining Supervisory Board Members or the sole remaining Supervisory Board Member shall be entrusted with the supervision of the management of the Company.

In the event that all Supervisory Board Members are absent or prevented from acting, two persons to be appointed for that purpose by the General Meeting, shall be temporarily entrusted with the supervision of the management of the Company.

**18. FINANCIAL YEAR, ANNUAL ACCOUNTS, ANNUAL REPORT**

18.1. Within five months after the end of each financial year, unless such period is extended by no more than six months by the General Meeting due to extraordinary circumstances, the Management Board shall prepare the annual accounts (consisting of the balance sheet and profit and loss account with explanatory notes thereto).

The annual accounts shall be signed by each Management Board Member and each Supervisory Board Member. If one or more of their signatures is missing, this fact and the reason thereof shall be stated.

Within the above-mentioned period, the Management Board shall prepare an annual report, unless the Company is not required to do so under the statutory rules and regulations applicable to the Company

18.2. If and to the extent that the Company is subject to the relevant legal provisions to this effect, the General Meeting shall instruct an auditor or a firm of auditors, as defined in article 2:393 paragraph 1 Dutch Civil Code, to audit the annual accounts and, if prepared, the annual report by the Management Board, to report thereon, and to issue an auditor's certificate with respect thereto.

A resolution of the General Meeting to appoint or dismiss the Company's auditor shall be adopted with a Qualified Shareholder Majority.

18.3. The annual accounts shall be adopted by a resolution of the General Meeting.

18.4. The Company is obliged to make its annual accounts, its annual report and the other information referred to in article 2:392 of the Dutch Civil Code publicly available at the trade register, if and to the extent required by law.

## **19. ALLOCATION OF PROFITS**

19.1. After having obtained the prior approval of the Class A Meeting, the General Meeting is authorised to resolve to distribute or allocate to the reserves (a part of) the profits, as these appear from the adopted annual accounts and/or to resolve to make interim distributions, including distributions from the reserves.

The Company may only make such distributions to its Shareholders to the extent that the Company's general equity exceeds the sum of the general equity which it is statutorily required to maintain.

19.2. Shares held by the Company in its own capital shall be disregarded for the calculation of the distribution of profits.

The Company may only make such distributions to its Shareholders to the extent that the Company's general equity exceeds the sum of the reserves which it is required to maintain by law and with due observance of the provisions of article 2: 216 Dutch Civil Code.

19.3. Any claim a Shareholder may have to a distribution shall lapse after five years, to be computed from the day on which such a distribution becomes payable.

## **20. GENERAL MEETINGS**

20.1. Within six months after the end of the financial year, the annual General Meeting shall be held.

20.2. The Management Board Members and Supervisory Board Members shall have an advisory vote in the General Meeting.

20.3. A General Meeting shall be convened by the Management Board, a Management Board Member, the Supervisory Board or a Supervisory Board Member.

20.4. The term 'right to attend meetings' in these articles of association is construed to mean the right to attend a General Meeting in person or to be represented in such meeting by a person holding a written proxy and the right to address such meeting. Shareholders and usufructuaries who have the voting rights have the right to attend meetings. The right to attend meetings is not attached to depositary receipts issued for shares.

20.5. The General Meetings shall be held in the municipality in which the company has its seat, in Naarden or in Haarlemmermeer (Schiphol).

20.6. A General Meeting may adopt valid resolutions, even in the event the legal formalities for convening and holding a General Meeting are disregarded, provided the persons having meeting rights as defined in article 2:227 Dutch Civil Code consent and the statutory requirements for adopting valid resolutions in such situations have been taken into account. Prior to the decision-making by the General Meeting, the Management Board Members and the Supervisory Board Members shall be allowed the opportunity to deliver their opinions.

20.7. Resolutions of the General Meeting shall be adopted by an Absolute Shareholder Majority unless these articles or Dutch stipulates otherwise. Blank votes shall be considered null and void.

The Management Board shall keep a record of all resolutions adopted.

Such record shall be available at the offices of the Company for inspection by the Shareholders and other persons with the right to attend meetings. Such persons shall be given a certified copy or an extract upon request, for a price which shall not exceed the costs.

20.8. If so determined by the Management Board and announced in the notice convening the meeting each Shareholder has the right to attend the General Meeting electronically, either in person or represented by a person holding a written proxy, to address such meeting and to exercise the voting right, provided that the use of the electronic means by such shareholder enables his identification, and enables such shareholder to take note of the discussions in the meeting directly, and to participate in the deliberations of the meeting.

- 20.9. The provisions of article 20.8 shall also apply to others entitled to attend meetings, provided however, that these persons cannot cast a vote if they are not entitled to do so.
- 20.10. For the application of articles 20.4 and 20.8, the requirement to have a written proxy is met if the proxy is granted electronically.
- 20.11. The Management Board is authorised to adopt regulations regarding the use of electronic means. If the Management Board has used its authority to adopt such regulations, these shall be made available at the time the meeting is convened.
- 20.12. If so announced at the time that the meeting is convened, votes can be cast electronically prior to the meeting, but no sooner than the thirtieth day prior to the day of the meeting.

**21. VOTES CAST IN SUCH WAY SHALL BE CONSIDERED EQUIVALENT TO THE VOTES CAST AT THE MEETING.**

In due observance of article 2:238 of the Dutch Civil Code Shareholders may also adopt resolutions without holding a General Meeting. The final sentence of article 20.6 applies accordingly.

**22. CLASS A MEETINGS, CLASS B MEETINGS, CLASS C MEETINGS.**

- 22.1. Class A Meetings, Class B Meetings and Class C Meetings shall be convened in accordance with article 20.3.
- 22.2. Resolutions of the Class A Meeting shall be adopted by an Absolute Class A Majority unless these articles or the law state otherwise.
- 22.3. Resolutions of the Class B Meeting shall be adopted by an Absolute Class B Majority unless these articles or the law state otherwise.
- 22.4. Resolutions of the Class C Meeting shall be adopted by an Absolute Class C Majority unless these articles or the law state otherwise.

- 22.5. The provisions of articles 20 and 21 shall apply accordingly.

**23. CONVERSION**

- 23.1. Class B Shares may be converted into Class A Shares pursuant to a resolution thereto adopted by the Management Board and with the prior written approval of the Class A Meeting, which resolution can only be adopted with a Qualified Class A Majority.
- 23.2. The holder of Class B Shares who intends to convert (part of) his Class B Shares into Class A Shares shall send a notice to the Management Board by registered mail indicating the number of Class B Shares he wishes to convert and the date of conversion, which date shall not be earlier than five (5) business days after the day the Management Board has received the notice, unless the holder of Class B Shares and the Management Board agree otherwise.
- 23.3. Each Class B Share can only be converted into one (1) Class A share as of the date of conversion referred to in article 23.2.
- 23.4. The Management Board shall make the appropriate registrations in the Shareholders register and filings with the trade register as soon as possible after the conversion.

**24. AMENDMENTS OF THE ARTICLES OF ASSOCIATION, LEGAL MERGER, DEMERGER, DISSOLUTION AND LIQUIDATION**

- 24.1. Subject to the provisions of these articles, the General Meeting may resolve to amend the articles of association, to a legal merger (juridische fusie), to a demerger (splittings) or to dissolve the Company.
- 24.2. In the event that a resolution to dissolve the Company is adopted, the liquidation shall be arranged by the Management Board, unless the General Meeting appoints other liquidators. In the resolution to dissolve the Company the remuneration to be paid to the liquidator or liquidators jointly shall also be determined.
- 24.3. During the liquidation these articles of association shall remain effective to the extent possible.
- 24.4. The surplus remaining after liquidation shall be distributed to the holders of Class A Shares, Class B Shares and Class C Shares in proportion to the number of Shares each owns.
- 24.5. Any GM Resolution referred to in this article can only be adopted with a Qualified Shareholder Majority.

DATED 19 APRIL 2012

Coöperatieve AAC LS U.A.

Forbion Co-Investment Coöperatief U.A.

Forbion Co-Investment II Coöperatief U.A.

Coöperatieve Gilde Healthcare II U.A.

and

uniQure B.V.

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**CLASS A SHAREHOLDERS  
AGREEMENT**

**relating to uniQure B.V.**

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SCHEDULE E (RESERVED MATTERS)

(ii)

**THIS CLASS A SHAREHOLDERS AGREEMENT** (the “**Agreement**”) is made on 19 April 2012.

**BETWEEN:**

- (1) **Coöperatieve AAC LS U.A.**, a coöperative (*coöperatie*) incorporated under the laws of the Netherlands with its corporate seat in Amsterdam, and its business address at Gooimeer 2 35, 1411 DC Naarden, the Netherlands and registered with the Commercial Register of Gooi-, Eem- en Flevoland under number 34256402 (the “**Existing Investor I**”);
- (2) **Forbion Co-Investment Coöperatief U.A.**, a coöperative (*coöperatie*) incorporated under the laws of the Netherlands with its registered office in Naarden, and its business address at Gooimeer 2 35, 1411 DC Naarden, the Netherlands and registered with the Commercial Register of Gooi-, Eem- en Flevoland under number 32142360 (the “**Existing Investor II**”);
- (3) **Forbion Co-Investment II Coöperatief U.A.**, a coöperative (*coöperatie*) incorporated under the laws of the Netherlands with its registered office in Naarden, and its business address at Gooimeer 2 35, 1411 DC Naarden, the Netherlands and registered with the Commercial Register of Gooi-, Eem- en Flevoland under number 53958713 (the “**New Investor I**”);
- (4) **Coöperatieve Gilde Healthcare II U.A.**, a coöperative (*coöperatie*) incorporated under the laws of the Netherlands with its corporate seat in Utrecht, and its business address at Newtonlaan 91 (3584 BP), the Netherlands and registered with the Commercial Register of Midden-Nederland under number 30216414 (the “**New Investor II**”);

and

- (5) **uniQure B.V.**, a private company with limited liability (*besloten vennootschap met beperkte aansprakelijkheid*), incorporated under the laws of the Netherlands with its registered office in Amsterdam, and its business address at Meibergdreef 61, 1105 BA Amsterdam Zuidoost, the Netherlands and registered with the Commercial Register of Amsterdam under number 54385229 (the “**Company**”);

and

- (6) any other holder from time to time of Ordinary Shares Class A in the capital of the Company.

The parties to this Agreement are hereinafter collectively referred to as the “**Parties**” and individually as a “**Party**”. Further details of the Parties are set out in SCHEDULE A (Parties). The Existing Investor I and the Existing Investor II are hereinafter jointly referred to as the “**Existing Investors**”. The New Investor I and the New Investor II are hereinafter jointly referred to as the “**New Investors**”. The Existing Investors and the New Investors, together with any other holder from time to time of Ordinary Shares Class A in the capital of the Company, are hereinafter collectively referred to as the “**Investors**” and individually as an “**Investor**”.

**RECITALS:**

- (A) The Existing Investors, the New Investor I, the Company and Amsterdam Molecular

Therapeutics (AMT) Holding N.V. (“**AMT**”) and its subsidiaries have entered into that certain “**Business Acquisition Agreement**” on 16 February 2012, pursuant to which the Company has agreed to acquire (and AMT has agreed to transfer) certain assets and liabilities of AMT in exchange for depository receipts (*certificaten van aandelen*) (each: a “**DR**” and any holder of a DR: a “**DR Holder**”) for Ordinary Shares Class B, issued without cooperation of the Company (*uitgegeven zonder medewerking van de vennootschap*) as referred to in Clause 2:227 paragraph 2 BW of the Dutch Civil Code by the Trust Foundation (the “**Transaction**”).

- (B) The Company is engaged in the development of human gene based therapies.
- (C) At the Completion Date, AMT has been dissolved and the DRs have been transferred to AMT and will be distributed to the AMT shareholders, whereupon each AMT shareholder that on the tenth Business Day following the date on which the dissolution and liquidation of AMT have come in effect (i.e. on or around 23 April 2012), holds at least 5% of the shares of AMT (each an “**Eligible DR Holder**”), is entitled (subject to the fulfilment of certain other conditions) to exchange its DRs for an equal number of Ordinary Shares Class A in the Company (the “**Exchange Offer**”).
- (D) Pursuant to the Business Acquisition Agreement, the Existing Investors and the New Investor I have committed to make an investment in an amount of in total EUR 6 million. Pursuant to the Existing Investors’ and the New Investor I’s commitment to provide equity funding to the Company, 9,771,986 Ordinary Shares Class A in the capital of the Company will be acquired by the Existing Investor II and the New Investor I at an issue price of EUR 0.614 per share on or around the date of this Agreement. In addition, AMT’s debts under the loan notes as assumed by the Company pursuant to the Transaction have been converted in 5,320,000 Ordinary Shares Class A in the capital of the Company issued to the Existing Investors at the Completion Date, at a conversion price of EUR 1.00 per share.
- (E) The New Investor II has committed to make an investment in an amount of in total EUR 1 million, pursuant to which commitment to provide equity funding to the Company, 1,628,664 Ordinary Shares Class A in the capital of the Company will be acquired by the New Investor II at an issue price of EUR 0.614 per share on around the date of this Agreement.
- (F) In this Agreement the Parties wish to set out the terms and conditions on which they have agreed to regulate the rights and obligations of the Investors with respect to the Company.

**IT IS AGREED** as follows:

## **1. INTERPRETATION**

1.1. In this Agreement, the following definitions are used:

“**Acceptance Deadline**” has the meaning given in Clause 4.5.2(D).

“**Acceptance Notice**” has the meaning given in Clause 4.5.3.

“**Accounting Policies**” means the specific principles, bases and conventions, rules and practices applied and used by the Company in preparing and presenting financial statements.

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“**Affiliate**” has the meaning given in article 2:24b of the DCC to the term “*groepsmaatschappij*” which, for the purposes of this Agreement, shall be deemed to include a Subsidiary, it being understood that a Shareholder shall not be considered an Affiliate of the Company, or of a Subsidiary of the Company.

“**Affiliated Fund**” means any investment company, limited partnership or person or entity of which the assets are managed professionally for investment purposes that has been, or may in the future be, established by the Investor or an Affiliate of an Investor and which is managed by an Investor or an Affiliate of an Investor.

“**Agreement**” means this shareholders agreement including schedules and appendices thereto as amended in accordance with its terms.

“**AMT**” has the meaning given in the recitals of this Agreement.

“**Applicable Accounting and Reporting Rules**” means (i) the Dutch Generally Accepted Accounting Principles (GAAP) or International Financial Reporting Standards (IFRS), as the case may be, or (ii) the relevant provisions on financial statements of the Dutch Civil Code and, to the extent applicable, the prevailing Guidelines for annual reporting in the Netherlands (*Richtlijnen voor de jaarverslaggeving*) published by the Dutch Accounting Standards Board (*Raad voor de Jaarverslaggeving*).

“**Articles of Association**” means the articles of association of the Company, as amended from time to time.

“**Asset Sale**” has the meaning given in Clause 5.1.2.

“**Business Day**” means a day (other than a Saturday or a Sunday) on which banks in the Netherlands are open for normal business.

“**Business Plan**” has the meaning given in Clause 6.1.3.

“**Business Acquisition Agreement**” means the business purchase agreement defined in the recitals of this Agreement.

“**Called Investors**” has the meaning given in Clause 4.9.1.

“**Called Shares**” has the meaning given in Clause 4.9.1.

“**Chairman**” has the meaning given in Clause 3.2.2.



“**Class A Meeting**” means the body corporate formed by the holders of Ordinary Shares Class A or the meeting of said body, as the case may be.

“**Company**” has the meaning given in the opening of this Agreement.

“**Completion**” means the consummation of the Transaction as contemplated in Clause 10 of the Business Acquisition Agreement.

“**Completion Date**” means 5 April 2012.

“**DCC**” means the Dutch Civil Code.

“**DR**” has the meaning as given in the recitals of this Agreement.

“**DR Holder**” has the meaning as given in the recitals of this Agreement.

“**Drag Along Notice**” has the meaning given in Clause 4.9.2.

“**Drag Along Option**” has the meaning given in Clause 4.9.1.

“**Eligible DR Holder**” has the meaning given in the recitals of this Agreement.

“**Employee Stock Option Plan**” has the meaning given in Clause 9.1.

“**Excess Sale Shares**” has the meaning given in Clause 4.5.2(F).

“**Exchange Offer**” has the meaning given in the recitals of this Agreement.

“**Existing Investor I**” has the meaning given in the opening of this Agreement.

“**Existing Investor II**” has the meaning given in the opening of this Agreement.

“**Existing Investors**” has the meaning given in the opening of this Agreement.

“**Exit**” has the meaning given in Clause 5.1.2.

“**Financial Year**” means the financial year of the Company as defined in the Articles of Association.

“**Financial Statements**” means the consolidated financial statements of the Company and the Subsidiaries and the separate financial statements of each Affiliate (to the extent applicable), including all notes, documents and statements thereto.

“**General Meeting**” means the general meeting of shareholders of the Company.

“**Group**” has the meaning given in Clause 3.1.4.

“**Group Company**” has the meaning given in Clause 3.1.4.

“**Independent Members**” has the meaning given in Clause 3.2.3.

“**Investor**” and “**Investors**” has the meaning given in the opening of this Agreement.

“**Investor Representatives**” has the meaning given in Clause 3.2.2.

“**Investor Shares**” means the Shares held by an Investor.

“**Listing**” means the admission of the Shares for trading on a regulated stock market.

“**Liquidation**” means a liquidation, dissolution or winding-up (*liquidatie of ontbinding*) of the Company.

“**Management Board**” means the management board (*raad van bestuur*) of the Company.

“**Management Board Member**” means a member of the Management Board.

“**New Investor I**” has the meaning given in the opening of this Agreement.

“**New Investor II**” has the meaning given in the opening of this Agreement.

“**New Investors**” has the meaning given in the opening of this Agreement.

“**Non-selling Investor**” and “**Non-selling Investors**” has the meaning given in Clause 4.5.1.

“**Ordinary Shares**” means the issued and outstanding from time to time Ordinary Shares Class A and the Ordinary Shares Class B.

“**Ordinary Shares Class A**” means the ordinary shares class A (*gewone aandelen*) in the capital of the Company with a nominal value of EUR 0.01 each.

“**Ordinary Shares Class B**” means the convertible ordinary shares class B (*gewone aandelen*) in the capital of the Company with a nominal value of EUR 0.01 each.

“**Original Purchase Price**” the purchase price of EUR 0.614 paid by the Existing Investor II and the New Investors for each Ordinary Share Class A consisting of the nominal value of EUR 0.01 plus an amount of EUR 0.604 as share premium (*agio*).

“**Portfolio Transfer**” has the meaning given in Clause 4.4.2.

“**Proposed Selling Investor**” and “**Proposed Selling Investors**” has the meaning given in Clause 4.5.1.

“**Qualified Majority**” means the affirmative vote of Shareholders representing at least 66 2/3 % of the Shares.

“**Sale**” has the meaning given in Clause 4.8.1.

“**Sale Shares**” has the meaning given in Clause 4.5.2(A).

“**Selling Investor**” or “**Selling Investors**” has the meaning given in Clause 4.8.1.

“**Shareholder**” means any holder of Shares.

“**Shares**” means the issued and outstanding shares from time to time in the capital of the Company, consisting of Ordinary Shares Class A and Ordinary Shares Class B.

“**Simple Majority Investor Consent**” means the affirmative vote of the Investors representing at least 51% of Ordinary Shares Class A.

“**Subsidiary**” has the meaning given in article 2:24a of the DCC to the term “*dochtermaatschappij*”.

“**Supervisory Board**” means the supervisory board (*raad van commissarissen*) of the Company.

“**Supervisory Board Member**” means a member of the Supervisory Board.

“**Supervisory Board Member A**” means a Supervisory Board Member appointed in accordance with Clause 3.2.2.

“**Supervisory Board Member B**” means a Supervisory Board Member appointed in accordance with Clause 3.2.3.

“**Tag Along Notice**” has the meaning given in Clause 4.8.1.

“**Tag Offer**” has the meaning given in Clause 4.8.3.

“**Third Party Purchaser**” has the meaning given in Clause 4.5.5.

“**Transaction**” means the transaction contemplated by the Business Acquisition Agreement.

“**Transfer Notice**” has the meaning given in Clause 4.5.1.

“**Trust Conditions**” means the trust conditions (*administratievoorwaarden*) adopted by the Trust Foundation, as amended from time to time.

“**Trust Foundation**” means Stichting Administratiekantoor uniQure B.V., a foundation organised under the laws of the Netherlands, having its registered office in Amsterdam Zuidoost, at Meibergdreef 61, 1105 BA, the Netherlands.

1.2. In this Agreement, unless otherwise specified:

- 1.2.1. the masculine gender shall include the feminine and the neuter and vice versa;
- 1.2.2. references to a person shall include a reference to any individual, company, association, partnership, trust or joint venture (in each case whether or not having separate legal personality);
- 1.2.3. references to “include” and “including” shall be treated as references to “include without limitation” or “including without limitation”;
- 1.2.4. unless the context requires otherwise, words in the singular shall include the plural and vice versa;
- 1.2.5. the headings are for identification only and shall not affect the interpretation of this Agreement.

## 2. SHARE CAPITAL

### 2.1. Authorised Share Capital

The authorised share capital of the Company at Completion is EUR 1,900,000 divided into 150,000,000 Ordinary Shares Class A and 40,000,000 Ordinary Shares Class B.

### 2.2. Issued Share Capital

- 2.2.1. Pursuant to the Business Acquisition Agreement 9,771,986 Ordinary Shares Class A have been or will be issued to or acquired by the Existing Investor II and the New Investor I in one or more tranches at an issue price of EUR 0.614 per

share on or around the date of this Agreement. In addition, pursuant to the Transaction, as a consequence of the conversion of the debt under the loan notes as assumed by the Company, 5,320,000 Ordinary Shares Class A have been issued to the Existing Investors at a conversion price of EUR 1,00 per share at the Completion Date.

- 2.2.2. Pursuant to the New Investor II's commitment to provide equity funding to the Company, 1,628,664 Ordinary Shares Class A in the capital of the Company will be issued to the New Investor II at an issue price of EUR 0.614 per share on or around the date of this Agreement.
- 2.2.3. The issued share capital of the Company after completion of the Transaction is as set out in SCHEDULE B (Share Capital).

## 2.3. Conversion of Ordinary Shares Class B

- 2.3.1. In accordance with Clause 23 of the Articles of Association, the Investors will resolve to approve a proposed resolution of the Management Board to convert the Ordinary Shares Class B underlying the DRs held by any Eligible DR Holder into an equal number of Ordinary Shares Class A in the Company pursuant to the Exchange Offer, provided that such Eligible DR Holder:
- (i) has irrevocably requested the termination from the Trust Foundation of the holding in trust of such number of Ordinary Shares Class B in accordance with Clause 6 of the Trust Conditions;
  - (ii) has entered into an Accession Agreement as set out in SCHEDULE C (Accession Agreement) to become a party to this Agreement as an Investor; and
  - (iii) holds, to be evidenced by a letter of an admitted institution (*aangesloten instelling*), (at least) an equal number of DRs at the time the request for termination as referred to above is made, as it held on the tenth Business Day following the Completion Date, as set out in Clause 6 of the Trust Conditions.
- 2.3.2. The Company shall not approve any demand to convert the Ordinary Shares Class B into Ordinary Shares Class A in the Company or adopt any resolution thereto, unless the conditions of Clause 2.3.1 have been fulfilled.

## 2.4. Dividend Policy

- 2.4.1. The Shareholders are entitled to the distribution of the profits of the Company for each Shareholder in proportion to the number of Shares that it holds.
- 2.4.2. The DR Holders are entitled to the distribution of the profits of the Company relating to the Ordinary Shares Class B held by the Trust Foundation, for each DR Holder in proportion to the number of DRs that it holds.
- 2.4.3. The Company shall apply profits available for distribution for its further development and expansion in accordance with the approved Business Plan prior to making any distributions to Shareholders.

# 3. CORPORATE GOVERNANCE

## 3.1. Management Board

- 3.1.1. The Management Board will at any time consist of at least two (2) Management Board Members appointed by the General Meeting in accordance with a binding nomination drawn up by the Class A Meeting in accordance with Clause 13.2 of the Articles of Association.
- 3.1.2. Immediately following Completion the Management Board of the Company shall consist of the directors constituting the AMT management board, i.e. Mr J. Aldag and Mr P.J. Morgan.
- 3.1.3. With regard to the Management Board Members nominated in accordance with Clause 3.1.1, the Investors will cast their vote in accordance with such nomination in or outside the General Meeting, as the case may be, in order to appoint the Management Board Member nominated by the Class A Meeting as member of the Management Board.
- 3.1.4. The Management Board will be responsible for all operational matters in respect of the Company and its Subsidiaries (collectively: the "Group" and any member of the Group also: a "Group Company").
- 3.1.5. Resolutions of the Management Board as set out in Part A of SCHEDULE D (Management Board and Supervisory Board) require the prior approval of the Supervisory Board. Such approval of the Supervisory Board can be obtained by a simple majority of the votes validly cast at a Supervisory Board meeting.
- 3.1.6. Resolutions of the Management Board as set out in Part B of SCHEDULE D (Management Board and Supervisory Board) require the prior approval of at least 51% of the holders of Ordinary Shares Class A.
- 3.1.7. Resolutions of the Management Board as set out in Part C of SCHEDULE D (Management Board and Supervisory Board) require the prior approval of a qualified majority of at least 66 2/3% of the holders of Ordinary Shares Class A.

- 3.1.8. A Management Board Member may be suspended and dismissed by the General Meeting. The Investors will cast their vote in relation to such proposed resolution to dismiss or suspend a Management Board Member in or outside the General Meeting, as the case may be, in accordance with a prior resolution of the Class A Meeting adopted thereto.
- 3.1.9. The remuneration of members of the Management Board shall be determined by the General Meeting and requires the prior approval of at least 51% of the holders of Ordinary Shares Class A, in accordance with Part B (Approval with Simple Majority Investor Consent (51%) of Ordinary Shares Class A held by Investor) of SCHEDULE E (Reserved Matters).
- 3.1.10. The Company shall maintain customary professional liability insurance for all members of the Management Board and the Supervisory Board.

### 3.2. Supervisory Board

- 3.2.1. The Supervisory Board shall consist of up to seven (7) Supervisory Board Members who shall have the title Supervisory Board Member A or Supervisory Board Member B, provided that at all times there shall be at least one Supervisory Board Member B more than there are Supervisory Board Members A.
- 3.2.2. The Supervisory Board Members A (one of whom shall be the chairman of the Supervisory Board (the “**Chairman**”)) will be appointed by the General Meeting in accordance with a binding nomination drawn up by the Class A Meeting in accordance with Clause 17.3 of the Articles of Association (the “**Investor Representatives**”).
- 3.2.3. The Supervisory Board Members B will be appointed by the General Meeting at its own discretion (the “**Independent Members**”).
- 3.2.4. Prior to the making of a nomination the Investor or group of Investors shall consult with the other Investor(s) and the Trust Foundation about the identity and the qualifications of such person included on a shortlist of potential nominees and the Investor or group of Investors shall take any substantiated objections against potential nominees into account in making their decision to formally nominate such person.
- 3.2.5. With regard to a Supervisory Board Member A nominated in accordance with Clause 3.2.2, the Investors will cast their vote in accordance with such nomination in or outside the General Meeting, as the case may be, in order to appoint the Supervisory Board Members A nominated by the Class A Meeting.
- 3.2.6. In case of a deadlock of votes in a meeting of the Supervisory Board, the Chairman will have a casting vote.
- 3.2.7. A Supervisory Board Member A that has been appointed in accordance with Clause 3.2.2, may be suspended and dismissed the General Meeting. The Investors will cast their vote in relation to such proposed resolution to dismiss or suspend a Supervisory Board Member A in or outside the General Meeting, as the case may be, in accordance with a prior resolution of the Class A Meeting adopted thereto.
- 3.2.8. Immediately following Completion the Supervisory Board will consist of S.J.H. van Deventer, H.A. Slootweg, as the Investor Representatives, and J.M. Feczko, P.M.M.J. van Holle, F. Meyer as the Independent Members.
- 3.2.9. The Supervisory Board shall meet at least six (6) times per year (or such other number as the Chairman may require) in person at scheduled meetings and so often as required for the proper fulfillment of the role of the Supervisory Board, either in person or by conference call.
- 3.2.10. The Supervisory Board will adopt resolutions by a simple majority of the votes cast. To be quorate, at least the majority of the Supervisory Board Members in office must be present or represented at a meeting of the Supervisory Board. In the event that such a quorum is not present or ceases to be present, the meeting is adjourned to the same day in the next week at the same time and place or at such time and place as determined by the members present at such meeting. If in any such adjourned meeting no quorum is present, the meeting may proceed and validly take the resolutions entered on the agenda of the first meeting. Votes may

also be rendered by written power of attorney given by one Supervisory Board Member to another Supervisory Board Member.

- 3.2.11. The Parties shall use their respective reasonable best efforts to ensure that any Supervisory Board meeting has the requisite quorum.
- 3.2.12. The Supervisory Board shall have at least two (2) standing committees, i.e. the audit committee and the remuneration and appointment committee, to be appointed by the Supervisory Board from its own members. Each committee shall consist of three (3) members. The Supervisory Board will appoint to each and every committee the most suitable Supervisory Board Members, provided that at least one (1) member of each and every committee will be an Investor Representative. Each committee shall have the right to draw up its internal regulations, subject to the approval of the Supervisory Board.

### 3.3. General Meeting

- 3.3.1. The Shareholders shall meet annually within six (6) months after the end of the Financial Year. Other meetings of the General Meeting may be held as often as necessary.
- 3.3.2. Unless provided otherwise in this Agreement, resolutions of the General Meeting shall be taken by a simple majority of the votes validly cast.
- 3.3.3. The Parties shall use their respective reasonable best efforts to ensure that any meeting of the General Meeting has the requisite quorum.

### 3.4. Reserved Matters

- 3.4.1. The matters referred to in Part A of SCHEDULE E (Reserved Matters) shall require a Qualified Majority.

3.4.2. The matters referred to in Part B of SCHEDULE E (Reserved Matters) shall require Simple Majority Investor Consent.

### 3.5. The Company's Affiliates

If any of the matters listed in SCHEDULE D (Management Board and Supervisory Board) and SCHEDULE E (Reserved Matters) relate to an Affiliate and are being dealt with at the level of such Affiliate, the Company shall procure that, in addition to the required approval of the appropriate corporate bodies of the Affiliate, as the case may be, such matters are made subject to the approval of the appropriate corporate bodies of the Company (as if such matters would relate to the Company rather than to the Affiliate), so that the Investors are able to, directly or indirectly through the respective corporate bodies of the Company, effectively exercise their consent rights in respect of such matters.

## 4. SHARES AND TRANSFERS

### 4.1. Anti-dilution

In the event of an issue of Shares at a price below the Original Purchase Price (other than Shares issued under the Employee Stock Option Plan), the Investors and the Company shall procure that Shares in the Company shall be issued either with payment to be made from

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the Company's share premium reserve, or if not available, such new Shares are to be issued at par value to the Existing Investor II and the New Investors, with the number of Shares so issued calculated according to the following formula:

$$(P1-P2) / P2 \times Q$$

Where:

- (i) P1 means the Original Purchase Price paid by the Existing Investor II and the New Investors;
- (ii) P2 means the issue price offered in the subsequent capital increase;
- (iii) Q means the number of Shares subscribed by the Existing Investor II and the New Investors (i.e. 9,771,986 plus 1,628,664).

### 4.2. No Encumbrance

An Investor is not entitled to directly or indirectly pledge (*verpanden*) or otherwise encumber (*bezwaren*) any Shares, or agree, whether conditionally or otherwise, to do any of the foregoing.

### 4.3. Prohibited Transfers

An Investor is not entitled to directly or indirectly transfer the economic or legal ownership of any Shares (including the issuance of a Transfer Notice pursuant to Clause 4.5.1), or agree, whether conditionally or otherwise, to do any of the foregoing, except:

- 4.3.1. with the prior written consent of the other Investors; or
- 4.3.2. where the transfer of Shares is required or permitted pursuant to this Agreement.

### 4.4. Permitted Transfers

Each Investor may, subject to the requirement for a transferee to enter into an Accession Agreement in accordance with Clause 9.4:

- 4.4.1. transfer free from any restrictions all or part of its Shares to an Affiliate or an Affiliated Fund;
- 4.4.2. transfer all or part of its Shares (other than to competitors of the Group) as part of a transfer of an Investor's investment portfolio (in whole or in part) (a "**Portfolio Transfer**").

### 4.5. Right of First offer other Investors

- 4.5.1. Except in the case of a transfer pursuant to Clause 4.4 (Permitted Transfers) of this Agreement, an Investor or group of Investors that wishes (or wish) to transfer any Shares (a "**Proposed Selling Investor**" or "**Proposed Selling Investors**") shall first give notice thereof in writing (the "**Transfer Notice**") to each other Investor (the "**Non-selling Investor**" or the "**Non-selling Investors**").
- 4.5.2. The Transfer Notice shall:

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- (A) specify the number of Shares the Proposed Selling Investor(s) wish(es) to sell (the "**Sale Shares**");
- (B) specify the name of the proposed transferee;
- (C) specify the minimum price (in cash or otherwise) at which it proposes to transfer the Sale Shares;
- (D) specify the period in which the offer shall remain open for acceptance, which period will lapse two (2) weeks from receipt of notice (the "**Acceptance Deadline**");

(E) be governed by the laws of the Netherlands;

(F) not be withdrawn by the Selling Investor before the Acceptance Deadline.

4.5.3. Upon receipt of the Transfer Notice, each of the Non-selling Investors is entitled to purchase such proportion of the Sale Shares as is equal to the proportion of the number of Shares held by it compared to the aggregate number of Shares held by all Non-selling Investors, in each case at the date of the Transfer Notice, for at least at the minimum price set out in the Transfer Notice or such other price as may be agreed between the Proposed Selling Investor(s) and the Non-selling Investor(s) by giving written notice (the “**Acceptance Notice**”) to the Proposed Selling Investor(s), before the Acceptance Deadline, on the basis that the Non-selling Investor(s) may accept all or part or none of the Sale Shares, provided that if a Non-selling Investor fails to give an Acceptance Notice before the Acceptance Deadline, it shall be deemed to have declined the offer made by the Proposed Selling Investor(s).

4.5.4. Any Non-selling Investor who accepts the offer made by the Proposed Selling Investor(s) before the Acceptance Deadline shall confirm in its Acceptance Notice either:

(A) that it would accept, on the same terms, the number of Sale Shares that have not been accepted by the other Non-selling Investor(s) before the Acceptance Deadline, if any, (the “**Excess Sale Shares**”); or

(B) that it would not accept any Excess Sale Shares,

provided that any such Non-selling Investor who fails to make such confirmation shall be deemed to have made a confirmation that it would not accept any Excess Sale Shares.

4.5.5. Any Excess Sale Shares shall be automatically allocated to each Non-selling Investor which has indicated that it will accept Excess Sale Shares, in proportion to the number of Shares held by such Non-selling Investor compared to the aggregate number of Shares held by all of the Non-selling Investors which have indicated that they would accept Excess Sale Shares, in each case at the date of the Transfer Notice, provided the Proposed Selling Investor(s) shall be entitled to transfer any Excess Sale Shares that have not been accepted by the Non-selling Investors pursuant to Clauses 4.5.4 and 4.5.5 and with due observance of Clause 4.5.7 to a third person and any other person acting in concert with that person (a “**Third Party Purchaser**”).

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4.5.6. Each Non-selling Investor which has accepted any Sale Shares pursuant to Clauses 4.5.3 and 4.5.5 shall be bound to buy such Sale Shares from the Proposed Selling Investor(s). In such event, completion of the sale and purchase of the Sale Shares shall take place within 20 Business Days after the Acceptance Deadline.

4.5.7. The Proposed Selling Investor(s) shall be entitled to transfer any Sale Shares that have not been accepted by the Non-selling Investor(s) pursuant to Clauses 4.5.3 up to and including 4.5.6 to the Third Party Purchaser at a price not less than the price, and on terms no more favourable than the terms, set out in the Transfer Notice, provided such sale is completed within 60 Business Days after expiry of the Acceptance Deadline. If so requested by the Non-selling Investor(s), the Proposed Selling Investor(s) shall provide evidence to prove that the transfer of the Sale Shares to the Third Party Purchaser occurred in accordance with this Clause 4.5. Any Third Party Purchaser shall be required to agree in writing (by signing an Accession Agreement as described in Clause 9.4) to be subject to and bound by the provisions of this Agreement.

#### 4.6. **General conditions Transfer**

Each transfer of Shares (be it to an Investor’s affiliate or group company, a Portfolio Transfer, a Sale or otherwise) is always subject to the condition that the purchaser of such transferred Shares is or becomes bound to this Agreement and other governing documents in accordance with Clause 9.4.

#### 4.7. **Lock-up Obligations**

During a period of two (2) years from Completion, the Investors may not sell any of their Shares in the Company unless if the provisions of the Clauses 4.4 (Permitted Transfers), 4.9 (Drag Along) or 5 (Exit) apply, or with the approval of an Investor or group of Investors holding 51% or more of the issued and outstanding Ordinary Shares Class A.

#### 4.8. **Tag Along**

4.8.1. If, after due observance of the relevant provisions of this Agreement, including but not limited to Clause 4.5 (Right of First Offer other Investors), an Investor or group of Investors holding 51% or more of the issued and outstanding Ordinary Shares Class A (the “**Selling Investor**” or “**Selling Investors**”), that wish(es) to sell any or all of the Shares held by it (or by them) to a Third Party Purchaser (a “**Sale**”), the Selling Investor(s) shall give not less than 15 Business Days advance notice of the proposed Sale to the Company and each other Investor and the Trust Foundation of the proposed Sale (a “**Tag Along Notice**”).

4.8.2. The Tag Along Notice shall:

(A) specify the number of Shares the Selling Investor(s) propose(s) to sell;

(B) specify the name of the Third Party Purchaser;

(C) specify the price (in cash or otherwise) per Share that the Third Party Purchaser is proposing to pay;

(D) specify the proposed date of transfer;

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- (E) be governed by the laws of the Netherlands;
- (F) specify the address where the counter notice should be sent.

4.8.3. As a condition to such Sale, the other Investors shall be entitled within 15 Business Days after receipt of the Tag Along Notice to notify the Selling Investor(s) that it wishes to sell a *pro rata parte* portion of the Shares held by it to the Third Party Purchaser at the proposed price by sending a counter notice to the address specified in the Tag Along Notice (the “**Tag Offer**”).

#### 4.9. Drag Along

- 4.9.1. In case a Selling Investor or Selling Investors, as the case may be, wish(es) to sell all, and for the avoidance of doubt, not less than all of the Shares held by it (or by them), to a Third Party Purchaser, the Selling Investor(s) shall have the right (the “**Drag Along Option**”), upon agreement on the terms and conditions of a bona fide offer by the Third Party Purchaser, to require all other Investors (the “**Called Investors**”) to sell and transfer all, and for the avoidance of doubt, not less than all, of their Shares (the “**Called Shares**”) to the proposed Third Party Purchaser in accordance with the provisions of this Clause 4.9.
- 4.9.2. The Selling Investor(s) that wish(es) to exercise its Drag Along Option shall give notice thereof in writing (the “**Drag Along Notice**”) to the Company and the Called Investors.
- 4.9.3. The transfer of the Called Shares to the Third Party Purchaser shall be completed within sixty (60) Business Days after service of the Drag Along Notice. The Called Investors shall be released from their obligation to transfer the Called Shares to the Third Party Purchaser if the Third Party Purchaser has not acquired the Called Shares on the expiration of such sixty (60) Business Day period.
- 4.9.4. If a Called Investor fails to cooperate with the transfer of its Called Shares to the Third Party Purchaser within a period of ten (10) Business Days from the proposed date of transfer, the Selling Investor(s) shall have the power and the duty to fulfil the obligations for and on behalf of the defaulting Called Investor.
- 4.9.5. Each Investor hereby grants an unconditional and irrevocable power of attorney to the Company to act on its behalf and to transfer the relevant Called Shares to the proposed purchaser in accordance with the provisions of this Clause 4.9.
- 4.9.6. The provisions of Clause 4.5 (Right of First Offer other Investors) and 4.8 (Tag Along) do not apply to a (proposed) transfer of Shares in respect of which a Drag Along Notice has been duly served pursuant to this Clause 4.9.

### 5. EXIT

#### 5.1. Asset Sale or Listing

- 5.1.1. It is the intention of the Parties that an Asset Sale or an Exit be achieved as soon as practically possible and commercially sensible.
- 5.1.2. An Exit (“**Exit**”) shall be:

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- a transfer (or a series of related transfers) of all the Shares issued and outstanding;
- the listing and admission to trading on a market for listed securities of either (i) the Shares, (ii) an intermediate holding company’s shares or (iii) the shares of new holding company established for the purposes of the Listing (a “**Listing**”); or
- a distribution pursuant to a winding-up or dissolution of the Company or any holding company of the Company, including following an Asset Sale.

5.1.3. An Asset Sale (“**Asset Sale**”) shall be a sale by one or more Group Companies of all, or substantially all, of the Group’s business, assets and undertaking.

5.1.4. A decision to achieve an Asset Sale requires the approval of the General Meeting and Simple Majority Investor Consent.

5.1.5. Each Party (taking into account its rights and obligations under this Agreement) shall take all steps that are required to ensure the success of any proposed Exit or Asset Sale, subject always to fiduciary duties and compliance with applicable law, including that:

- (A) each Investor shall dispose of (a *pro rata parte* portion of) its Shares on the same terms and conditions as the other Investors;
- (B) each of the Investors shall on a Listing, retain such number of Shares held at the time of the Listing for such period after the Listing as is required by the relevant listing rules or is recommended by the Company’s financial advisors in such Listing;
- (C) the Company shall procure that each of the Management Board Members shall continue to work for the Company on the same terms and conditions for a period of one (1) year after an Exit if so requested by one or more Investors;
- (D) in the event of a Listing on a US stock market, the Investors shall be entitled to appropriate registration rights on terms to be agreed.

#### 5.2. Forced Exit

If after six (6) years following Completion neither an Asset Sale nor an Exit has been achieved, the Investors holding 51% or more of the issued and outstanding Ordinary Shares Class A will at any time have the right to require the other Investors and the Company to cooperate in an Exit or Asset Sale.

### 5.3. Appointment of Advisors

Investors holding 51% or more of the issued and outstanding Ordinary Shares Class A and the Company shall jointly decide on the appointment by the Company of its advisors in connection with the Exit.

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## 6. FINANCIAL REPORTING

### 6.1. Provision of Information

6.1.1. The Company shall in addition to the statutory obligations prepare and deliver to the Investors:

- at least 30 Business Days prior to the start of the new Financial Year, a draft annual budget (for comment) including profit and loss projections, monthly cash flow projections and balance sheet projections including line items on total proposed (i) capital expenditure, (ii) project financing and (iii) total costs of other investments for comments by the Investors;
- within 30 days after the start of the relevant Financial Year, the annual budget per the above specifications;
- within 15 Business Days after the end of each month, a monthly information package to include (i) updated liquidity forecast (12 months, in aggregate and breakdown per major project) and reconciliation with previous month, (ii) qualitative comments highlighting development progress, financing events and sales process, and (iii) progress reporting on 5 (to the extent applicable) biggest projects (format to be acceptable to Investors holding 51% or more of the issued and outstanding Ordinary Shares Class A, but in any case shall include executive summary on key elements (progress, costs, liquidity against budget etc));
- within 20 Business Days after the end of each quarter, a quarterly information package to include (i) monthly information, (ii) updated format revised Business Plan as stipulated in Clause 6.1.3; (iii) a reconciliation with previous quarterly revised business plan; (iv) progress reporting on 5 (to the extent applicable) biggest projects (format to be acceptable to Investors holding 51% or more of the issued and outstanding Ordinary Shares Class A (acting reasonably) but in any case shall include: (a) executive summary on key elements (progress, costs, liquidity against budget, project financing, general economical climate etc) and (b) project monitor summary if project has commenced (to be delivered in the same format as required by the financiers of the relevant project, if available) and (v) reporting on financing (format to be acceptable to Investors holding 51% or more of the issued and outstanding Ordinary Shares Class A (acting reasonably) but shall in any case include regularity of all financings);
- as soon as they become available, copies of annual valuations of underlying assets; and
- other information reasonably required by Investors holding 51% or more of the issued and outstanding Ordinary Shares Class A.

6.1.2. The format of the reporting shall be designed by the Company and shall be in form and substance satisfactory to Investors holding 51% or more of the issued and outstanding Ordinary Shares Class A.

6.1.3. The business plan (“**Business Plan**”) as referred to in Clause 6.1.1 includes:

- (i) a business forecast with respect to the next Financial Year;

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- (ii) the proposed business strategy of the Company;

- (iii) details of the assumptions used to prepare the information in (i) and (ii); and

- (iv) a budget, including:

- (a) a breakdown of expected monthly revenues, operating expenses, operating results, net interest expenses and net profits, capital expenditures and cash flow;
- (b) a projected balance sheet and profit and loss account as per the end of the next Financial Year;
- (c) an overview of expected funding requirements, including the proposed sourcing of such funding;

6.1.4. The financial information referred to in Clause 6.1.1 shall be:

- (A) prepared in accordance with the Applicable Accounting and Reporting Rules and the Accounting Policies, in each case consistently applied;
- (B) in English;
- (C) expressed in Euro.

6.1.5. The Management Board shall procure that the Company and its Subsidiaries shall be managed in accordance with the provisions of the budget and the Business Plan. The Management Board shall keep true and accurate books of account and records in accordance with sound accounting practices, employing standards, procedures and forms in conformity with mandatory requirements of the applicable law and shall procure that the management of the Subsidiaries of the Company shall do the same.



## **7. CONFIDENTIALITY**

- 7.1. Subject to Clause 7.2, each Party shall treat as strictly confidential and not disclose or use any information relating to this Agreement or any ancillary matter and the negotiations leading up to this Agreement and including the disclosure or use of any information relating to the Group and its business operations.
- 7.2. The restrictions contained in Clause 7.1 shall not apply if and to the extent:
- (A) disclosure is required by any law or by a court;
  - (B) disclosure is required by any securities exchange or regulatory or governmental body;
  - (C) disclosure is necessary to enforce this Agreement in court proceedings.
  - (D) the other Parties have given their written consent to disclosure;
  - (E) the information has come into the public domain through no fault of the relevant

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Party's group;

- (F) disclosure is necessary to obtain the advice of any professional adviser;
- (G) disclosure is necessary within the relevant Party's group.

In the event of a disclosure of information pursuant to Clause 7.2 (A) or (B), the disclosing Party shall consult with the other Parties (to the extent permitted by applicable laws or regulations) as to the contents, form and timing of the disclosure to be made.

- 7.3. The restrictions contained in this Clause 7.1 shall apply to each Party (as applicable) during the term of this Agreement and shall remain in full force and effect after an Investor ceases to be an Investor.

## **8. TERM AND TERMINATION**

### **8.1. Term**

This Agreement shall remain in full force and effect from the date of Completion until terminated in accordance with the terms of Clause 8.2.

### **8.2. Termination**

- 8.2.1. This Agreement can be terminated by unanimous consent of all Parties in writing.
- 8.2.2. This Agreement shall automatically terminate upon:
- (A) completion of an Exit or Asset Sale;
  - (B) completion of a Liquidation;
  - (C) acquisition by one Investor of all Shares.
- 8.2.3. This Agreement shall terminate in respect of a Shareholder from the date it ceases to be a Shareholder.

### **8.3. Surviving Clauses**

Termination of this Agreement shall be without prejudice to:

- 8.3.1. any right, liability or obligation accrued under this Agreement but not satisfied or discharged at the date of termination; and
- 8.3.2. the provisions of the Clauses 7 (Confidentiality), 9.5 (Notices), 9.15 (Governing Law) and 9.17 (Jurisdiction), which will remain in full force and effect.

## **9. MISCELLANEOUS**

### **9.1. Employee Stock Option Plan**

- 9.1.1. To provide further incentives to employees, directors and or outside consultants and advisors, contemporary with completion of the Transaction, the Investors will authorise a new unallocated pool of options and/or warrants for future grants of

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DRs issued by the Trust Foundation to employees, directors and/or outside consultants and advisors, representing 15% of the fully diluted share capital of the Company post Completion, with:

- (i) a vesting period of three years; 1/3 of the options to be vested after one year, the other 2/3 to be vested in year 2 and 3 on a pro rata, i.e. linear basis; and

(ii) the exercise price of the options being EUR 0.614 per DR (the “**Employee Stock Option Plan**”).

9.1.2. The Supervisory Board shall be authorised to grant options or warrants within the scheme. A resolution of the Supervisory Board to this effect requires the positive vote of at least two Investor Representatives.

9.1.3. SCHEDULE B (Share Capital) shall be updated upon an issue of Ordinary Shares Class B to the Trust Foundation in relation to the Employee Stock Option Plan, to properly reflect the relevant changes.

## 9.2. **Further Assurances**

Each Investor shall exercise or refrain from exercising, as the case may be, all voting rights attached to its Shares and waive any pre-emption rights and other rights it may have under the Articles of Association and exercise or refrain from exercising, as the case may be, all other powers of control available to it in relation to the Company so as to procure (to the extent possible) that at all times during the term of this Agreement the provisions of this Agreement are duly and promptly observed and given full force and effect according to their spirit and intention.

## 9.3. **Conflict**

Subject to applicable law, in case of an ambiguity or a conflict between provisions of the Articles of Association and provisions of this Agreement, the provisions of this Agreement shall prevail.

## 9.4. **Additional Investors**

9.4.1. No issue or transfer of Ordinary Shares Class A to any person who is not a Party to this Agreement shall be effectuated without first obtaining from such person a duly signed Accession Agreement in the form of SCHEDULE C (Accession Agreement), provided that the Parties to this Agreement will procure and will cooperate to sign such Accession Agreement in respect of any person that is permitted to acquire Ordinary Shares Class A pursuant to this Agreement and to whom it is envisaged to transfer such Ordinary Shares Class A.

9.4.2. SCHEDULE A (Parties) and SCHEDULE B (Share Capital) shall be updated upon an issue or transfer of Shares to properly reflect the relevant changes.

## 9.5. **Large Company Regime**

If at any time during the term of this Agreement the Company falls under the scope of the articles 2:262 through 2:274 of the DCC (*verplicht structuurregime*), Parties will use their

best efforts to ensure that the corporate governance of the Company will be structured to the maximum extent possible in accordance with the spirit and intention of this Agreement.

## 9.6. **Notices**

All notices, consents, waivers and other communications under this Agreement must be in writing in English and delivered by hand or sent by registered mail, express courier, fax or e-mail to such addresses and fax numbers as a Party may notify to the other Parties from time to time. A notice shall be effective upon receipt and shall be deemed to have been received at the time of delivery, if delivered by hand, registered mail or express courier, or at the time of successful transmission, if delivered by fax or e-mail.

## 9.7. **Amendment**

This Agreement may only be amended by unanimous consent of all Parties in writing.

## 9.8. **Assignment**

None of the Parties may assign or procure the assumption of its rights and obligations under this Agreement, either in whole or in part, to any other person without the prior written consent of the other Parties, provided that an Investor may assign the whole or part of its rights under this Agreement to any person who has acquired Shares from the Investor in accordance with Clause 4.4.1 and 4.4.2.

## 9.9. **Entire Agreement**

This Agreement constitutes the entire agreement between the Parties with respect to the subject matter of this Agreement. This Agreement supersedes any and all earlier agreements, either verbally or in writing, between the Parties in relation to the subject matter of this Agreement.

## 9.10. **Partial Invalidity**

The invalidity or unenforceability of any provision of this Agreement shall not affect the validity or enforceability of any other provision of this Agreement. Any such invalid or unenforceable provision shall be replaced or be deemed to be replaced by a provision that is considered to be valid and enforceable. The interpretation of the replacing provisions shall be as close as possible to the intent of the invalid or unenforceable provision.

## 9.11. **Compensation of costs**

Each of the Parties hereto shall pay its own expenses incurred or to be incurred in connection with this Agreement and matters incidental to this Agreement, except for the expenses incurred or to be incurred by the Existing Investors and the New Investor I, which expenses shall be borne by the Company.

## 9.12. **No Waiver**

9.13. **No Rescission**

The Parties waive their right to rescind (*ontbinden*) this Agreement pursuant to article 2:265 DCC after Completion.

9.14. **Counterparts**

This Agreement may be signed in any number of counterparts each of which, when executed by one or more of the Parties, shall constitute an original.

9.15. **Governing Law**

This Agreement is governed by the laws of the Netherlands.

9.16. **Accounting regime**

The Dutch GAAP will apply. The audit committee will be authorised to adopt IFRS as the applicable accounting standard.

9.17. **Jurisdiction**

All disputes arising in connection with this Agreement shall be finally settled in accordance with the rules of the Netherlands Arbitration Institute (*Nederlands Arbitrage Instituut*) and:

- 9.17.1. the arbitral tribunal shall be composed of three arbitrators;
- 9.17.2. the place of arbitration will be Amsterdam, the Netherlands;
- 9.17.3. the language of the proceedings will be English (unless the Parties agree otherwise);
- 9.17.4. the arbitrators will decide according to the rules of Dutch law;
- 9.17.5. the arbitral award will be final and binding;
- 9.17.6. to ensure that the arbitral award shall not be published, each Party shall notify the administrator of the NAI within one calendar month after receipt of the arbitral award that they object to publication of the arbitral award by the NAI;
- 9.17.7. the proceedings shall not be consolidated with other arbitral proceedings pursuant to Article 1046 of the Dutch Code of Civil Procedure.

[signature pages to follow]

**This Agreement has been entered into on the date first above written.**

**Coöperatieve AAC LS U.A.**

/s/ Signature Illegible  
By: Forbion 1 Management B.V.  
  
Title: Director  
  
By: Name Illegible  
Title: Partner

**Forbion Co-Investment Coöperatief U.A.**

/s/ Signature Illegible  
By: Forbion 1 Management B.V.  
  
Title: Director  
  
By: Name Illegible  
Title: Partner

**Forbion Co-Investment II Coöperatief U.A.**

/s/ Signature Illegible  
By: Forbion 1 Co II Management B.V.  
  
Title: Director

**Coöperatieve AAC LS U.A.**

/s/ Signature Illegible  
By: Forbion 1 Management B.V.  
  
Title: Director  
  
By: Name Illegible  
Title: Partner

**Forbion Co-Investment Coöperatief U.A.**

/s/ Signature Illegible  
By: Forbion 1 Management B.V.  
  
Title: Director  
  
By: Name Illegible  
Title: Partner

**Forbion Co-Investment II Coöperatief U.A.**

/s/ Signature Illegible  
By: Forbion 1 Co II Management B.V.  
  
Title: Director

By: Name Illegible  
Title: Partner

By: Name Illegible  
Title: Partner

## Coöperatieve Gilde Healthcare II U.A.

/s/ Edwin de Graaf

By: Gilde Healthcare II Management B.V.

Title: Director

By: /s/ Edwin de Graaf  
Title: Partner

## uniQure B.V.

/s/ Aldag

By:  
Title: Director

## uniQure B.V.

/s/ PJ Morgan

By: PJ Morgan  
Title: Director

## SCHEDULE A (PARTIES)

- (1) **Coöperatieve AAC LS U.A.**, a coöperative (*coöperatie*) incorporated under the laws of the Netherlands with its corporate seat in Amsterdam, and its business address at Gooimeer 2 35, 1411 DC Naarden, the Netherlands and registered with the Commercial Register of Gooi-, Eem- en Flevoland under number 34256402, represented by its managing director **Forbion 1 Management B.V.**, a private company with limited liability (*besloten vennootschap met beperkte aansprakelijkheid*), incorporated under the laws of the Netherlands with its registered office (*zetel*) in Amsterdam, and its business address at Gooimeer 2 35, 1411 DC Naarden, the Netherlands and registered with the Commercial Register of Gooi-, Eem- en Flevoland under number 34249898);
- (2) **Forbion Co-Investment Coöperatief U.A.**, a coöperative (*coöperatie*) incorporated under the laws of the Netherlands with its registered office in Naarden, and its business address at Gooimeer 2 35, 1411 DC Naarden, the Netherlands and registered with the Commercial Register of Gooi-, Eem- en Flevoland under number 32142360, represented by its managing director **Forbion 1 Management B.V.**, a private company with limited liability (*besloten vennootschap met beperkte aansprakelijkheid*), incorporated under the laws of the Netherlands with its registered office (*zetel*) in Amsterdam, and its business address at Gooimeer 2 35, 1411 DC Naarden, the Netherlands and registered with the Commercial Register of Gooi-, Eem- en Flevoland under number 34249898);
- (3) **Forbion Co-Investment II Coöperatief U.A.**, a coöperative (*coöperatie*) incorporated under the laws of the Netherlands with its registered office in Naarden, and its business address at Gooimeer 2 35, 1411 DC Naarden, the Netherlands and registered with the Commercial Register of Gooi-, Eem- en Flevoland under number 53958713, represented by its managing director **Forbion 1 CO II Management B.V.**, a private company with limited liability (*besloten vennootschap met beperkte aansprakelijkheid*), incorporated under the laws of the Netherlands with its registered office (*zetel*) in Amsterdam, and its business address at Gooimeer 2 35, 1411 DC Naarden, the Netherlands and registered with the Commercial Register of Gooi-, Eem- en Flevoland under number 53951956);
- (4) **Coöperatieve Gilde Healthcare II U.A.**, a coöperative (*coöperatie*) incorporated under the laws of the Netherlands with its corporate seat in Utrecht, and its business address at Newtonlaan 91 (3584 BP), the Netherlands and registered with the Commercial Register of Midden-Nederland under number 30216414, represented by its managing director **Gilde Healthcare II Management B.V.**, a private company with limited liability (*besloten vennootschap met beperkte aansprakelijkheid*), incorporated under the laws of the Netherlands with its registered office (*zetel*) seat in Utrecht, and its business address at Newtonlaan 91 (3584 BP), the Netherlands and registered with the Commercial Register of Midden-Nederland under number 30215056;
- (5) **uniQure B.V.**, a private company with limited liability (*besloten vennootschap met beperkte aansprakelijkheid*), incorporated under the laws of the Netherlands with its registered office in Amsterdam, and its business address at Meibergdreef 61, 1105 BA Amsterdam Zuidoost, the Netherlands and registered with the Commercial Register of Amsterdam under number 54385229, duly represented by its managing directors Mr. J. Aldag and Mr. P. Morgan.

## SCHEDULE B (SHARE CAPITAL)

### PART 1: Share Capital after completion of the Transaction

Shareholder	Number of Ordinary Shares Class A	Number of Ordinary Shares Class B	% Outstanding
Coöperatieve AAC LS U.A.	4,575,200		9.6%
Forbion Co-Investment Coöperatief U.A.	1,189,317		2.5%
Forbion Co-Investment II Coöperatief U.A.	9,327,469		19.5%
Coöperatieve Gilde Healthcare II U.A.	1,628,664		3.4%
Stichting Administratiekantoor uniQure B.V.		31,101,065	65.0%

## SCHEDULE C (ACCESSION AGREEMENT)

This Accession Agreement (the “**Agreement**”) is made on [•]

**RECITALS:**

- (A) [Note: insert name] (the “**New Shareholder**”) on [Note: insert date] acquired [Note: insert number] Ordinary Shares Class A by [an issue of new Shares] [a transfer of Shares by [Note: insert name of transferor] the “**Transferor**”)] [an issue of new Ordinary Shares Class A pursuant to conversion of Ordinary Shares Class B].
- (B) This Agreement is entered into in compliance with the terms of the class A shareholders agreement dated [Note: insert date] between the Existing Investors, the New Investors and the Company (all as defined therein) (which agreement is referred to in this Agreement as the “**Class A Shareholders Agreement**”).

**IT IS AGREED** as follows:

- (1) Definitions used in this Agreement have the same meaning as given to them in the Class A Shareholders Agreement unless stated otherwise and the provisions of Clause 1 (Interpretation) of the Class A Shareholders Agreement shall apply to this Agreement.
- (2) The New Shareholder agrees to become a Party to the Class A Shareholders Agreement and to be bound by the terms of the Class A Shareholders Agreement in all respects as an Investor.
- (3) SCHEDULE A (Parties) to the Class A Shareholders Agreement shall be updated to properly reflect the relevant changes in the ownership of the Shares.
- (4) [Note: insert other relevant details]
- (5) The provisions of the Clauses 9.6, 9.7, 9.8, 9.9, 9.10, 9.14, 9.15 and 9.17 of the Class A Shareholders Agreement shall apply *mutatis mutandis* to this Agreement.

**THUS AGREED AND SIGNED ON [•],**

[Note: to be signed by New Shareholder and Parties to the Class A Shareholders Agreement]

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**SCHEDULE D (MANAGEMENT BOARD AND SUPERVISORY BOARD)**

**Part A**

**Supervisory Board approval**

- (a) Any amendment of articles of association of any Group Company.
- (b) Voting on shares or similar equity interests in a Group Company (which provision would need to be mirrored in articles of association of Group Companies), for resolutions mentioned in Part A, B and C.
- (c) The instigation or the settlement of any material litigation or arbitration or mediation proceedings by a Group Company, for the purpose of which ‘material’ shall mean an interest or claim that is of strategic importance to the Group or has a monetary value of at least EUR 100,000.
- (d) Any proposals to the General Meeting to materially change the emoluments of members of the Management Board, including bonuses and option schemes.
- (e) The removal or appointment of the auditors of any Group Company, other than the reappointment of existing auditors.
- (f) Remuneration of the auditors of the Company.
- (g) Approval of any change in accounting policies of any Group Company.
- (h) Alteration to the financial year end of any Group Company.
- (i) Non-project related capital expenditure exceeding in one transaction or event or a series of related transactions or events, in excess of an amount of EUR 50,000 but less than EUR 100,000, which is not included in an approved business plan or budget.

**Part B**

**Approval with Simple Majority Investor Consent (51%) of Class A Ordinary Shares held by Investors**

- (a) Unless specified in an approved business plan of the Company, entering into or materially changing borrowing and lending arrangements (including issuance of debt instruments) by any Group Company, exceeding an amount of EUR 250,000.
- (b) Unless specified in an approved business plan of the Company, establishing/closing any material branch, establishment, agency or business of any Group Company.
- (c) Unless specified in an approved business plan of the Company, entering into any material joint venture, partnership or profit sharing arrangement or licensing agreement by any Group Company.
- (d) Unless specified in an approved business plan of the Company, the expansion or development of the Group or any of its business other than through a Group Company.

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- (e) Adoption of or amendment to the current business plan (to be in agreed form) and budget (to be in agreed form).
  - (f) Creation or release of any security or (save in the ordinary course of trading and consistent with past practice) granting of guarantees by any Group Company, exceeding an amount of EUR 250,000.
  - (g) Unless specified in an approved business plan of the Company, any material acquisitions or disposals by any Group Company.
  - (h) The appointment or removal of any member of the Supervisory Board or Managing Director of a Group Company other than the Company.
  - (i) Non-project related capital expenditure exceeding in one transaction or event or a series of related transactions or events, in an amount of EUR 100,000 or more, which is not included in an approved business plan or budget.
  - (j) Establishment and material amendment of any management incentive scheme of any Group Company (other than the Company).

### **Part C**

#### **Approval with Qualified Majority Investor Consent (66 2/3%) of Class A Ordinary Shares held by Investors**

- (a) Any change in a Group Company's (other than the Company's) share capital.
  - (b) Unless specified in an approved business plan of the Company, any material change of the nature or the name of the business of the Group.
  - (c) Entry into, termination or variation of any contract or arrangement by a Group Company with an Investor, other than financing arrangements.
  - (d) Any distribution from reserves (other than wholly intra-group) by any Group Company.
  - (e) Transactions by a Group Company outside of its ordinary course.
  - (f) Taking steps to commence insolvency or winding-up proceedings of a Group Company (including the application for suspension of payment of debts by a Group Company).
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## **SCHEDULE E (RESERVED MATTERS)**

### **Part A**

#### **Approval with Qualified Shareholder Consent**

The following resolutions of the General Meeting will require the affirmative vote of Shareholders holding at least 66<sup>2</sup>/<sub>3</sub>% of Ordinary Shares.

#### **Approval with Qualified Majority Shareholder Consent (66 2/3%) of Ordinary Shares**

- (a) The merger (fusie) or demerger (splitsing) of the Company.
- (b) The initiation of liquidation or dissolution of the Company or approve the filing for bankruptcy.
- (c) The amendment of the articles of association of the Company.
- (d) Appointment or dismissal of the Company's auditors.

### **Part B**

#### **Approval with Simple Majority Investor Consent (51%) of Class A Ordinary Shares held by Investors**

The following resolutions of the General Meeting will require the affirmative vote of Investors holding at least 51% of Class A Ordinary Shares.

- (a) The issue of new equity securities (including options and warrants).
- (b) The exclusion or restriction of pre-emptive rights with respect to the issue of new equity securities.
- (c) The redemption (*intrekking*) or the reduction of the nominal value of any shares.
- (d) The purchase (*inkoop*) by the Company of shares in its own capital, shares in the capital of any subsidiary, or depositary receipts (*certificaten van aandelen*) representing any such shares (whether or not issued "with the co-operation of the Company").
- (e) The declaration of dividends or distributions.
- (f) The delegation of powers with respect to the issue of securities, the exclusion of pre-emptive rights, or the approval of the purchase of the Company's own shares.
- (g) Determination or variation of the remuneration of members of the Management Board and of the Supervisory Board.



## ACCESSION AGREEMENT CLASS A SHAREHOLDERS AGREEMENT

This Accession Agreement (the “**Agreement**”) is made on 25 May 2012 and made between:

- (1) **COÖPERATIEVE AAC LS U.A.**, a coöperative (*coöperatie*) incorporated under the laws of the Netherlands with its corporate seat in Amsterdam, and its business address at Gooimeer 2 35, 1411 DC Naarden, the Netherlands and registered with the Commercial Register of Gooi-, Eem- en Flevoland under number 34256402 (the “**Existing Investor I**”);
- (2) **FORBION CO-INVESTMENT COÖPERATIEF U.A.**, a coöperative (*coöperatie*) incorporated under the laws of the Netherlands with its registered office in Naarden, and its business address at Gooimeer 2 35, 1411 DC Naarden, the Netherlands and registered with the Commercial Register of Gooi-, Eem- en Flevoland under number 32142360 (the “**Existing Investor II**”);
- (3) **FORBION CO-INVESTMENT II COÖPERATIEF U.A.**, a coöperative (*coöperatie*) incorporated under the laws of the Netherlands with its registered office in Naarden, and its business address at Gooimeer 2 35, 1411 DC Naarden, the Netherlands and registered with the Commercial Register of Gooi-, Eem- en Flevoland under number 53958713 (the “**New Investor I**”);
- (4) **COÖPERATIEVE GILDE HEALTHCARE II U.A.**, a coöperative (*coöperatie*) incorporated under the laws of the Netherlands with its corporate seat in Utrecht, and its business address at Newtonlaan 91 (3584 BP), the Netherlands and registered with the Commercial Register of Midden-Nederland under number 30216414 (the “**New Investor II**”);
- (5) **UNIQUE B.V.**, a private company with limited liability (*besloten vennootschap met beperkte aansprakelijkheid*), incorporated under the laws of the Netherlands with its registered office in Amsterdam, and its business address at Meibergdreef 61, 1105 BA Amsterdam Zuidoost, the Netherlands and registered with the Commercial Register of Amsterdam under number 54385229 (the “**Company**”);
- (6) **S.J.H. VAN DEVENTER C.V.**, a Dutch limited partnership (*commanditaire vennootschap*), having its registered address at 1411 DC Naarden, Gooimeer 2 35, registered in the trade register under number 32158843 (the “**New Shareholder 1**”);
- (7) **CREDIT LYONNAIS INNOVATION 6**, a French venture capital fund (*Fonds Commun de Placement dans l’Innovation*), having its business location at 100 Boulevard de Montparnasse, 75014 Paris, France (the “**New Shareholder 2**”), which is represented by its managing company the French limited liability company (*société anonyme à directoire et conseil de surveillance*) OMNES Capital, incorporated and existing under the laws of France, having its registered address at 100 Boulevard du Montparnasse, 75014 Paris, France and registered with the French Registry of Commerce and Companies under number 428 711 196 TCS Paris (“**OMNES Capital**”);
- (8) **LCL INNOVATION 1**, a French venture capital fund (*Fonds commun de placement dans l’innovation*), having its business location at 100 Boulevard de Montparnasse, 75014 Paris, France (the “**New Shareholder 3**”), which is represented by OMNES Capital;
- (9) **CLVC**, a French corporation (*Société anonyme*), incorporated and existing under the laws of France, having its registered office at 100 Boulevard du Montparnasse, 75014 Paris, France, and registered with the French Registry of Commerce and Companies under number 434.465.514 RCS Paris, France (the “**New Shareholder 4**”), which is represented by OMNES Capital;
- (10) **CLV 1**, a French *Fonds Commun de Placement à Risques*, having its business location at 100 Boulevard de Montparnasse, 75014 Paris, France (the “**New Shareholder 5**”), which is represented by OMNES Capital;
- (11) **ADVENT PRIVATE EQUITY FUND IV**, an English partnership, incorporated and existing under the laws of the United Kingdom, having its registered address at 25 Buckingham Gate, London, SW1E 6LD, United Kingdom and registered in England and Wales under number LP10002 (the “**New Shareholder 6**”);
- (12) **ADVENT MANAGEMENT IV LP**, a Scottish limited partnership, incorporated and existing under the laws of Scotland, having its registered address at 50 Lothian Road, Festival Square, Edinburgh, EH3 9WJ, United Kingdom and registered in Scotland under number SL005366 (the “**New Shareholder 7**”);
- (13) **TIVERINA INVERSIONES S.L.**, a Spanish private company with limited liability, having its place of business at Calle Zurbano nº76 6ª, Madrid, Spain, with Tax Identification Number B-85381960 (the “**New Shareholder 8**”);
- (14) **ABADAI INVERSIONES S.L.**, a Spanish private company with limited liability, having its place of business at Calle Zurbano nº76 6ª, Madrid, Spain, with Tax Identification Number B-85383511 (the “**New Shareholder 9**”);
- (15) **SURRIC INVERSIONES S.L.**, a Spanish private company with limited liability, having its place of business at Calle Zurbano nº76 6ª, Madrid, Spain, with Tax Identification Number B-85381507 (the “**New Shareholder 10**”);
- (16) **HILOS Y POLO S.L.**, a Spanish private company with limited liability, having its place of business at Calle Zurbano nº76 6ª, Madrid, Spain, with Tax Identification Number B-83914481 (the “**New Shareholder 11**”);
- (17) **RAMON MORA-FIGUEROA MORA-FIGUEROA**, with professional domicile at Madrid, Calle Zurbano nº76 6ª and holder of a National Identity Card with number 31.241.827-F (the “**New Shareholder 12**”);
- (18) **ACTIVOS Y TENENCIAS 85 S.L.**, a Spanish private company with limited liability, having its place of business at Calle Ana Teresa 85 B, Madrid Spain, with Tax identification number B-84366111 (the “**New Shareholder 13**”);
- (19) **FUNDACION PARA EL DESARROLLO Y LA COOPERACION INTERNACIONAL**, having its place of business at Calle Jose Abascal nº44 2º, Madrid, Spain, with Tax identification number G-80787161 (the “**New Shareholder 14**”);



- (20) **FUNDACION UNIVERSITARIA DE NAVARRA**, having its place of business at Avd. Pio XII 53 1º, Pamplona, Spain, with Tax Identification Number G-31469125 (the “**New Shareholder 15**”);

- (21) **JOSE LUIS PASCUAL PLAZA**, with professional domicile at Madrid, Calle del Pastor 4 and holder of a National Identity Card with number 02.153.208-V (the “**New Shareholder 16**”);
- (22) **LUPUS ALPHA MICRO CHAMPIONS**, a Sub Fund of Lupus alpha Fonds, a Fonds Commun de placement under Luxembourg law, for which and on behalf wherof Lupus alpha Investment S.A., 69, route d’Esch, L-1470 Luxembourg, R.C.S. Luxembourg B-79272 (Management Company) acts (the “**New Shareholder 17**”); and
- (23) **LUPUS ALPHA ALL OPPORTUNITIES FUND**, a Sub Fund of Lupus alpha Fonds, a Fonds Commun de placement under Luxembourg law, for which and on behalf wherof Lupus alpha Investment S.A., 69, route d’Esch, L-1470 Luxembourg, R.C.S. Luxembourg B-79272 (Management Company) acts (the “**New Shareholder 18**”),

The parties to this Agreement are hereinafter collectively referred to as the “**Parties**” and individually as a “**Party**”. The New Shareholders 1 up to and including 18 collectively referred to as the “**New Shareholders**” and individually as a “**New Shareholder**”.

**RECITALS:**

- (A) By an issue of new Ordinary Shares Class A pursuant to conversion of Ordinary Shares Class B following the Exchange Offer:

- (i) The New Shareholder 1 will acquire 49,298 Ordinary Shares Class A;
- (ii) The New Shareholder 2 will acquire 576,520 Ordinary Shares Class A;
- (iii) the New Shareholder 3 will acquire 512,462 Ordinary Shares Class A;
- (iv) the New Shareholder 4 will acquire 401,320 Ordinary Shares Class A;
- (v) the New Shareholder 5 will acquire 69,716 Ordinary Shares Class A;
- (vi) the New Shareholder 6 will acquire 3,724,371 Ordinary Shares Class A;
- (vii) the New Shareholder 7 will acquire 37,241 Ordinary Shares Class A;
- (viii) the New Shareholder 8 will acquire 478,084 Ordinary Shares Class A;
- (ix) the New Shareholder 9 will acquire 210,824 Ordinary Shares Class A;
- (x) the New Shareholder 10 will acquire 210,824 Ordinary Shares Class A;
- (xi) the New Shareholder 11 will acquire 66,000 Ordinary Shares Class A;
- (xii) the New Shareholder 12 will acquire 48,000 Ordinary Shares Class A;
- (xiii) the New Shareholder 13 will acquire 31,764 Ordinary Shares Class A;
- (xiv) the New Shareholder 14 will acquire 269,768 Ordinary Shares Class A;

- (xv) the New Shareholder 15 will acquire 230,631 Ordinary Shares Class A;
- (xvi) the New Shareholder 16 will acquire 10,000 Ordinary Shares Class A;
- (xvii) the New Shareholder 17 will acquire 1,560,000 Ordinary Shares Class A; and
- (xviii) the New Shareholder 18 will acquire 850,000 Ordinary Shares Class A.

- (B) This Agreement is entered into in compliance with the terms of the class A shareholders agreement dated 19 April 2012 between the Existing Investors, the New Investors and the Company (which agreement is referred to in this Agreement as the “**Class A Shareholders Agreement**”).

**IT IS AGREED** as follows:

- (1) Definitions used in this Agreement have the same meaning as given to them in the Class A Shareholders Agreement unless stated otherwise and the provisions of Clause 1 (Interpretation) of the Class A Shareholders Agreement shall apply to this Agreement.
- (2) With effect from the date of this Agreement, the New Shareholder becomes a Party to and will be bound by the terms of the Class A Shareholders Agreement in all respects as an Investor, under the condition precedent (*opschortende voorwaarde*) that the New Shareholder has acquired the Ordinary Shares Class A pursuant to the execution of the Exchange Offer as referred to in Recital A above.

- (3)

SCHEDULE A (Parties) to the Class A Shareholders Agreement shall be updated to properly reflect the relevant changes in the ownership of the Shares.
- (4)

The provisions of the Clauses 9.6, 9.7, 9.8, 9.9, 9.10, 9.14, 9.15 and 9.17 of the Class A Shareholders Agreement shall apply *mutatis mutandis* to this Agreement.

THUS AGREED AND SIGNED ON 25 May 2012

[signature pages to follow]

This Agreement has been entered into on the date first above written.

Coöperatieve AAC LS U.A.

/s/ H. A. Slootweg  
By: \_\_\_\_\_  
  
Title: Director  
By: H. A. Slootweg  
Title: Director

Forbion Co-Investment Coöperatief U.A.

/s/ H. A. Slootweg  
By: Forbion 1 Management B.V.  
  
Title: Director  
By: H. A. Slootweg  
Title: Director

Forbion Co-Investment II Coöperatief U.A.

/s/ H. A. Slootweg  
By: Forbion 1 Co II Management B.V.  
  
Title: Director  
By: H. A. Slootweg  
Title: Director

Coöperatieve Gilde Healthcare II U.A.

/s/ Edwin de Graaf  
By: Gilde Healthcare II Management B.V.  
  
Title: Director  
By: Edwin de Graaf  
Title: Managing Director

uniQure B.V.

/s/ Aldag  
By: Aldag  
Title: Director

Coöperatieve AAC LS U.A.

/s/ M. A van Osch  
By: Forbion 1 Management B.V.  
  
Title: Director  
By: M. A van Osch  
Title: Director

Forbion Co-Investment Coöperatief U.A.

/s/ M. A van Osch  
By: Forbion 1 Management B.V.  
  
Title: Director  
By: M. A van Osch  
Title: Director

Forbion Co-Investment II Coöperatief U.A.

/s/ M. A van Osch  
By: Forbion 1 Co II Management B.V.  
  
Title: Director  
By: M. A van Osch  
Title: Director

uniQure B.V.

/s/ PJ Morgan  
By: PJ Morgan  
Title: Director

This Agreement has been entered into on the date first above written.

S.J.H. Van Deventer C.V.

/s/ H. A. Slootweg  
By: Forbion Capital Partners Management Services B.V.  
  
Title: General Partner  
By: H. A. Slootweg  
Title: Director

S.J.H. Van Deventer C.V.

/s/ M. A van Osch  
By: Forbion Capital Partners Management Services B.V.  
  
Title: General Partner  
By: M. A van Osch  
Title: Director

This Agreement has been entered into on the date first above written.

Credit Lyonnais Innovation 6

/s/ Fabien Prévost

By: OMNES capital

Title: Managing company  
By: Fabien Prévost  
Title: Title Illegible

LCL Innovation 1

/s/ Fabien Prévost

By: OMNES capital

Title: Managing company  
By: Fabien Prévost  
Title: Title Illegible

CLVC

/s/ Fabien Prévost

By: Fabien Prévost  
Title: Title Illegible

CLV 1

/s/ Fabien Prévost

By: OMNES capital

Title: Managing company  
By: Fabien Prévost  
Title: Title Illegible

This Agreement has been entered into on the date first above written.

Advent Private Equity Fund IV LP

/s/ Signature Illegible

By: Advent Management IV Limited Partnership acting as General Partner by Advent Venture Partners LLP acting as Manager

By: Name Illegible  
Title: Partner

Advent Management IV Limited Partnership

/s/ Signature Illegible

By: Advent Venture Partners LLP

By: Name Illegible  
Title: Partner

Advent Private Equity Fund IV LP

/s/ Signature Illegible

By: Advent Management IV Limited Partnership acting as General Partner by Advent Venture Partners LLP acting as Manager

By: Name Illegible  
Title: Partner

Advent Management IV Limited Partnership

/s/ Signature Illegible

By: Advent Venture Partners LLP

By: Name Illegible  
Title: Partner

This Agreement has been entered into on the date first above written.

Tiverina Inversiones S.L

Tiverina Inversiones S.L

/s/ Signature Illegible

By: Name Illegible

Title: Director

By: Name Illegible

Title:

Abadai Inversiones S.L

/s/ Signature Illegible

By: Name Illegible

Title: Director

By: Name Illegible

Title:

Surric Inversiones S.L

/s/ Signature Illegible

By: Name Illegible

Title: Director

By: Name Illegible

Title:

Hilos y Polo S.L

/s/ Signature Illegible

By: Name Illegible

Title: Director

By:

Title:

N/A

By:

Title: Director

By:

Title:

Abadai Inversiones S.L

N/A

By:

Title: Director

By:

Title:

Surric Inversiones S.L

N/A

By:

Title: Director

By:

Title:

Hilos y Polo S.L

N/A

By:

Title: Director

By:

Title:

This Agreement has been entered into on the date first above written.

Ramon Mora-Figueroa Mora-Figueroa

/s/ Signature Illegible

Activos y Tenencias 85 S.L

/s/ Signature Illegible

By: Name Illegible

Title: Director

By:

Title:

Fundacion para el Desarrollo y la Cooperación Internacional

/s/ Signature Illegible

By: Name Illegible

Title: Director

By:

Title:

Jose Luis Pascual Plaza

/s/ Signature Illegible

Acitvos y Tenencias 85 S.L

N/A

By:

Title: Director

By:

Title:

Fundacion para el Desarrollo y la Cooperación Internacional

/s/ Signature Illegible

By: Name Illegible

Title: Director

By:

Title:

/s/ Signature Illegible

By:

Title: Director

By: Name Illegible

Title:

/s/ Signature Illegible

By:

Title: Director

By: Name Illegible

Title:

This Agreement has been entered into on the date first above written.

Lupus Alpha Micro Champions

/s/ Signature Illegible /s/ Signature Illegible

By: Lupus alpha Investment S.A.

Title: Management Company

By: Name Illegible Name Illegible

Title: MD MD

Lupus Alpha Micro Champions

/s/ Signature Illegible /s/ Signature Illegible

By: Lupus alpha Investment S.A.

Title: Management Company

By: Name Illegible Name Illegible

Title: MD MD

Lupus Alpha All Opportunities Fund

By: Lupus alpha Investment S.A.

Title: Management Company

By: Name Illegible Name Illegible

Title: MD MD

Lupus Alpha All Opportunities Fund

By: Lupus alpha Investment S.A.

Title: Management Company

By: Name Illegible Name Illegible

Title: MD MD

DATED 19 APRIL 2012

Coöperatieve AAC LS U.A.

Forbion Co-Investment Coöperatief U.A.

Forbion Co-Investment II Coöperatief U.A.

Coöperatieve Gilde Healthcare II U.A.

and

Stichting Administratiekantoor uniQure B.V.

and

uniQure B.V.

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**CLASS B SHAREHOLDERS AGREEMENT**

**relating to uniQure B.V.**

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SCHEDULE A (PARTIES)

SCHEDULE B (ACCESSION AGREEMENT)

BETWEEN:

- (1) **Coöperatieve AAC LS U.A.**, a coöperative (*coöperatie*) incorporated under the laws of the Netherlands with its corporate seat in Amsterdam, and its business address at Gooimeer 2 35, 1411 DC Naarden, the Netherlands and registered with the Commercial Register of Gooi-, Eem- en Flevoland under number 34256402 (the “**Existing Investor I**”);
- (2) **Forbion Co-Investment Coöperatief U.A.**, a coöperative (*coöperatie*) incorporated under the laws of the Netherlands with its registered office in Naarden, and its business address at Gooimeer 2 35, 1411 DC Naarden, the Netherlands and registered with the Commercial Register of Gooi-, Eem- en Flevoland under number 32142360 (the “**Existing Investor II**”);
- (3) **Forbion Co-Investment II Coöperatief U.A.**, a coöperative (*coöperatie*) incorporated under the laws of the Netherlands with its registered office in Naarden, and its business address at Gooimeer 2 35, 1411 DC Naarden, the Netherlands and registered with the Commercial Register of Gooi-, Eem- en Flevoland under number 53958713 (the “**New Investor I**”);
- (4) **Coöperatieve Gilde Healthcare II U.A.**, a coöperative (*coöperatie*) incorporated under the laws of the Netherlands with its corporate seat in Utrecht, and its business address at Newtonlaan 91 (3584 BP), the Netherlands and registered with the Commercial Register of Midden-Nederland under number 30216414 (the “**New Investor II**”);
- (5) **Stichting Administratiekantoor uniQure B.V.**, a foundation organised under the laws of the Netherlands, having its registered office in Amsterdam, at Meibergdreef 61, 1105 BA Amsterdam Zuidoost, the Netherlands and registered with the Commercial Register of Amsterdam under number 55055036 (the “**Trust Foundation**”);

and

- (6) **uniQure B.V.**, a private company with limited liability (*besloten vennootschap met beperkte aansprakelijkheid*), incorporated under the laws of the Netherlands with its registered office in Amsterdam, and its business address at Meibergdreef 61, 1105 BA Amsterdam Zuidoost, the Netherlands and registered with the Commercial Register of Amsterdam under number 54385229 (the “**Company**”);

and

- (7) any other holder from time to time of Ordinary Shares Class A in the capital of the Company.

The parties to this Agreement are hereinafter collectively referred to as the “**Parties**” and individually as a “**Party**”. Further details of the Parties are set out in SCHEDULE A (Parties). The Existing Investor I and the Existing Investor II are hereinafter jointly referred to as the “**Existing Investors**”. The New Investor I and the New Investor II are hereinafter jointly referred to as the “**New Investors**”. The Existing Investors and the New Investors, together with any other holder from time to time of Ordinary Shares Class A in the capital of the Company, are hereinafter collectively referred to as the “**Investors**” and individually as an “**Investor**”.

---

RECITALS:

- (A) The Existing Investors, the New Investor I, the Company and Amsterdam Molecular Therapeutics (AMT) Holding N.V. (“**AMT**”) and its subsidiaries have entered into that certain “**Business Acquisition Agreement**” on 16 February 2012, pursuant to which the Company has agreed to acquire (and AMT has agreed to transfer) certain assets and liabilities of AMT in exchange for depositary receipts (*certificaten van aandelen*) (each: a “**DR**” and any holder of a DR: a “**DR Holder**”) for Ordinary Shares Class B, issued without cooperation of the Company (*uitgegeven zonder medewerking van de vennootschap*) as referred to in Clause 2:227 paragraph 2 BW of the Dutch Civil Code by the Trust Foundation (the “**Transaction**”).
- (B) The Company is engaged in the development of human gene based therapies.
- (C) In this Agreement the Parties wish to set out the terms and conditions on which they have agreed to regulate the rights and obligations of the Trust Foundation with respect to the ordinary shares Class B it holds from time to time.

IT IS AGREED as follows:

1. INTERPRETATION

- 1.1. In this Agreement, the following definitions are used:

“**Agreement**” means this shareholders agreement including schedules and appendices thereto as amended in accordance with its terms.

“**AMT**” has the meaning given in the recitals of this Agreement.

“**Articles of Association**” means the articles of association of the Company, as amended from time to time.

“**Asset Sale**” has the meaning given in Clause 4.2.

“**Business Day**” means a day (other than a Saturday or a Sunday) on which banks in the Netherlands are open for normal business.

“**Business Acquisition Agreement**” has the meaning given in the recitals of this Agreement.

“**Called Shareholders**” has the meaning given in Clause 3.3.1.

“**Called Shares**” has the meaning given in Clause 3.3.1.

“**Company**” has the meaning given in the opening of this Agreement.

“**DCC**” means the Dutch Civil Code.

“**DR**” has the meaning given in the recitals of this Agreement.

“**DR Holder**” has the meaning given in the recitals of this Agreement.

“**Drag Along Notice**” has the meaning given in Clause 3.3.2.

“**Drag Along Option**” has the meaning given in Clause 3.3.1.

“**Existing Investor I**” has the meaning given in the opening of this Agreement.

“**Existing Investor II**” has the meaning given in the opening of this Agreement.

“**Existing Investors**” has the meaning given in the opening of this Agreement.

“**Exit**” has the meaning given in Clause 4.2.

“**Group**” means the Company together with its Subsidiaries.

“**Investor**” and “**Investors**” has the meaning given in the opening of this Agreement.

“**Listing**” has the meaning given in Clause 4.2.

“**Liquidation**” means a liquidation, dissolution or winding-up (*liquidatie of ontbinding*) of the Company.

“**New Investor I**” has the meaning given in the opening of this Agreement.

“**New Investor II**” has the meaning given in the opening of this Agreement.

“**New Investors**” has the meaning given in the opening of this Agreement.

“**Ordinary Shares Class A**” means the ordinary shares class A (*gewone aandelen*) in the capital of the Company with a nominal value of EUR 0.01 each.

“**Ordinary Shares Class B**” means the convertible ordinary shares class B (*gewone aandelen*) in the capital of the Company with a nominal value of EUR 0.01 each.

“**Sale**” has the meaning given in Clause 3.2.1.

“**Selling Investor**” and “**Selling Investors**” has the meaning given in Clause 3.2.1.

“**Shareholder**” means any holder of Shares.

“**Shares**” means the issued and outstanding shares from time to time in the capital of the Company, consisting of Ordinary Shares Class A and Ordinary Shares Class B.

“**Subsidiary**” has the meaning given in article 2:24a of the DCC to the term “*dochtermaatschappij*”.

“**Tag Along Notice**” has the meaning given in Clause 3.2.1.

“**Tag Offer**” has the meaning given in Clause 3.2.3.

“**Third Party Purchaser**” has the meaning given in Clause 3.2.1.

“**Transaction**” has the meaning given in the recitals of this Agreement.

“**Trust Conditions**” means the trust conditions (*administratievoorwaarden*) adopted by the Trust Foundation, as amended from time to time.

“**Trust Foundation**” has the meaning given in the opening of this Agreement.

1.2. In this Agreement, unless otherwise specified:

1.2.1. the masculine gender shall include the feminine and the neuter and vice versa;

1.2.2. references to a person shall include a reference to any individual, company, association, partnership, trust or joint venture (in each case whether or not having separate legal personality);



- 1.2.3. references to “include” and “including” shall be treated as references to “include without limitation” or “including without limitation”;
- 1.2.4. unless the context requires otherwise, words in the singular shall include the plural and vice versa;
- 1.2.5. the headings are for identification only and shall not affect the interpretation of this Agreement.

## 2. DIVIDEND POLICY

- 2.1.1. The Shareholders are entitled to the distribution of the profits of the Company for each Shareholder in proportion to the number of Shares that it holds.
- 2.1.2. The DR Holders are entitled to the distribution of the profits of the Company relating to the Ordinary Shares Class B held by the Trust Foundation, for each DR Holder in proportion to the number of DRs that it holds.
- 2.1.3. The Company shall apply profits available for distribution for its further development and expansion prior to making any distributions to Shareholders.

## 3. TRANSFERS OF SHARES

### 3.1. General conditions Transfer

- 3.1.1. The Trust Foundation shall not be authorised to transfer any of the Shares it holds, unless provided otherwise in this Agreement.
- 3.1.2. The DRs are freely transferable, subject to restrictions under applicable laws.

### 3.2. Tag Along

- 3.2.1. If an Investor or group of Investors holding 51% or more of the issued and outstanding Ordinary Shares Class A (the “**Selling Investor**” or “**Selling Investors**” as the case may be), that wishes (or wish) to sell any or all of the Shares held by it (or by them) to a person or any other person acting in concert with that person (a “**Third Party Purchaser**”) (a “**Sale**”), the Selling Investor(s) shall give not less than 15 Business Days advance notice of the proposed Sale to the Company and the Trust Foundation (a “**Tag Along Notice**”).
- 3.2.2. The Tag Along Notice shall:
  - (A) specify the number of Shares the Selling Investor(s) propose(s) to sell;

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- (B) specify the name of the Third Party Purchaser;
- (C) specify the price (in cash or otherwise) per Share that the Third Party Purchaser is proposing to pay;
- (D) specify the proposed date of transfer;
- (E) be governed by the laws of the Netherlands;
- (F) specify the address where the counter notice should be sent.

- 3.2.3. As a condition to such Sale, the Trust Foundation shall be entitled within 15 Business Days after receipt of the Tag Along Notice to notify the Selling Investor(s) that they wish to sell a *pro rata parte* portion of the Shares held by it at the proposed price by sending a counter notice to the address specified in the Tag Along Notice (the “**Tag Offer**”).

### 3.3. Drag Along

- 3.3.1. In case a Selling Investor or Selling Investors, as the case may be, wish(es) to sell all, and for the avoidance of doubt, not less than all of the Shares held by it (or by them), to a Third Party Purchaser, the Selling Investor(s) shall have the right (the “**Drag Along Option**”), upon agreement on the terms and conditions of a bona fide offer by a Third Party Purchaser, to require all other Shareholders (the “**Called Shareholders**”) to sell and transfer all, and for the avoidance of doubt, no less than all, of their Shares (the “**Called Shares**”) to the proposed Third Party Purchaser in accordance with the provisions of this Clause 3.3.
- 3.3.2. The Selling Investor(s) that wish(es) to exercise the Drag Along Option shall give notice thereof in writing (the “**Drag Along Notice**”) to the Company and the Called Shareholders.
- 3.3.3. The transfer of the Called Shares to the Third Party Purchaser shall be completed within sixty (60) Business Days after service of the Drag Along Notice. The Called Shareholders shall be released from their obligation to transfer the Called Shares to the Third Party Purchaser if the Third Party Purchaser has not acquired the Called Shares on the expiration of such sixty (60) Business Day period.
- 3.3.4. If a Called Shareholder fails to cooperate with the transfer of its Called Shares to the Third Party Purchaser within a period of ten (10) Business Days from the proposed date of transfer, the Selling Investor(s) shall have the power and the duty to fulfil the obligations for and on behalf of the defaulting Called Shareholder.
- 3.3.5. Each Shareholder hereby grants an unconditional and irrevocable power of attorney to the Company to act on its behalf and to transfer the relevant Called Shares to the proposed purchaser in accordance with the provisions of this Clause 3.3.
- 3.3.6. The provisions of Clause 3.2 (Tag Along) do not apply to a (proposed) transfer of Shares of which a Drag Along Notice has been duly served pursuant to this Clause 3.3.

#### 4. ASSET SALE OR EXIT

- 4.1. It is the intention of the Parties that an Asset Sale or an Exit be achieved as soon as practically possible and commercially sensible.
- 4.2. An Exit (“**Exit**”) shall be:
- a transfer (or a series of related transfers) of all the Shares issued and outstanding;
  - the listing and admission to trading on a market for listed securities of either (i) the Shares, (ii) an intermediate holding company’s shares or (iii) the shares of new holding company established for the purposes of the Listing (a “**Listing**”); or
  - a distribution pursuant to a winding-up or dissolution of the Company or any holding company of the Company, including following an Asset Sale.
- 4.3. An Asset Sale (“**Asset Sale**”) shall be a sale by the Company and/or one or more of its Subsidiaries of all, or substantially all, of the Group’s business, assets and undertaking.
- 4.4. A decision to achieve an Asset Sale requires the approval of the general meeting of shareholders of the Company and the affirmative vote of the Investors representing at least 51% of Ordinary Shares Class A.
- 4.5. Each Party (taking into account its rights and obligations under this Agreement) shall take all steps that are required to ensure the success of any proposed Exit or Asset Sale, subject always to fiduciary duties and compliance with applicable law, including that:
- (A) each Shareholder shall dispose of (a *pro rata parte* portion of) its Shares on the same terms and conditions as the other Shareholders; and
  - (B) each of the Shareholders shall on a Listing, retain such number of Shares held at the time of the Listing for such period after the Listing as is required by the relevant listing rules or is recommended by the Company’s financial advisors in such Listing.

#### 5. CONFIDENTIALITY

- 5.1. Subject to Clause 5.2, each Party shall treat as strictly confidential and not disclose or use any information relating to this Agreement or any ancillary matter and the negotiations leading up to this Agreement and including the disclosure or use of any information relating to the Group and its business operations, unless provided otherwise in this Agreement and provided that a copy of this Agreement shall be available at the offices of the Trust Foundation.
- 5.2. The restrictions contained in Clause 5.1 shall not apply if and to the extent:
- (A) disclosure is required by any law or by a court;
  - (B) disclosure is required by any securities exchange or regulatory or governmental body;
  - (C) disclosure is necessary to enforce this Agreement in court proceedings.

- (D) the other Parties have given their written consent to disclosure;
- (E) the information has come into the public domain through no fault of the relevant Party’s group;
- (F) disclosure is necessary to obtain the advice of any professional adviser;
- (G) disclosure is necessary within the relevant Party’s group.

In the event of a disclosure of information pursuant to Clause 5.2 (A) or (B), the disclosing Party shall consult with the other Parties (to the extent permitted by applicable laws or regulations) as to the contents, form and timing of the disclosure to be made.

- 5.3. The restrictions contained in this Clause 5.1 shall apply to each Party (as applicable) during the term of this Agreement and shall remain in full force and effect after a Shareholder ceases to be a Shareholder.

#### 6. TERM AND TERMINATION

##### 6.1. Term

This Agreement shall remain in full force and effect from the date of Completion until terminated in accordance with the terms of Clause 6.2.

##### 6.2. Termination

- 6.2.1. This Agreement can be terminated by unanimous consent of all Parties in writing.
- 6.2.2. This Agreement shall automatically terminate upon:

- (A) completion of an Exit or Asset Sale;
- (B) completion of a Liquidation;
- (C) acquisition by one Shareholder of all Shares;
- (D) disposal by the Trust Foundation of all the Shares it holds.

6.2.3. This Agreement shall terminate in respect of a Shareholder from the date it ceases to be a Shareholder.

## 6.3. **Surviving Clauses**

Termination of this Agreement shall be without prejudice to:

- 6.3.1. any right, liability or obligation accrued under this Agreement but not satisfied or discharged at the date of termination; and
- 6.3.2. the provisions of the Clauses 5 (Confidentiality), 7.4 (Notices), 7.13 (Governing Law) and 7.14 (Jurisdiction), which will remain in full force and effect.

## 7. **MISCELLANEOUS**

### 7.1. **Trust Foundation Information Rights**

- 7.1.1. Within 20 Business Days after a general meeting of shareholders of the Company, the DR Holders shall be entitled to receive from the management board of the Trust Foundation a copy of the adopted annual accounts of the Company and a list of resolutions adopted by the general meeting of shareholders of the Company.
- 7.1.2. Within 20 Business Days after the end of each quarter, the DR Holders shall be entitled to receive from the management board of the Trust Foundation a quarterly financial statement of the Company, in a format to be approved by the supervisory board of the Company.
- 7.1.3. There shall be no obligation to make the information as referred to in this Clause 7.1 publicly available by placing it on the Company's or any other website.

### 7.2. **Conflict**

Subject to applicable law, in case of an ambiguity or a conflict between provisions of the Articles of Association and provisions of this Agreement, the provisions of this Agreement shall prevail.

### 7.3. **Additional Investors**

- 7.3.1. No issue or transfer of Ordinary Shares Class A to any person who is not a Party to this Agreement shall be effectuated without first obtaining from such person a duly signed Accession Agreement in the form of SCHEDULE B (Accession Agreement), provided that the Parties to this Agreement will procure and will cooperate to sign such Accession Agreement in respect of any person that is permitted to acquire Ordinary Shares Class A pursuant to this Agreement and to whom it is envisaged to transfer such Ordinary Shares Class A.
- 7.3.2. The Parties to this Agreement will procure and will cooperate that any party who has converted Ordinary Shares Class B underlying the DRs held by it into an equal number of Ordinary Shares Class A pursuant to the Transaction and in accordance with Clause 6 of the Trust Conditions and Clause 23 of the Articles of Association, will become a party to this Agreement as an Investor by duly signing an Accession Agreement in the form of SCHEDULE B (Accession Agreement).
- 7.3.3. SCHEDULE A (Parties) shall be updated upon an issue or transfer of Shares to properly reflect the relevant changes.

### 7.4. **Notices**

All notices, consents, waivers and other communications under this Agreement must be in writing in Dutch or in English and delivered by hand or sent by registered mail, express courier, fax or e-mail to such addresses and fax numbers as a Party may notify to the other Parties from time to time. A notice shall be effective upon receipt and shall be deemed to have been received at the time of delivery, if delivered by hand, registered mail or express courier, or at the time of successful transmission, if delivered by fax or e-mail.

### 7.5. **Amendment**

This Agreement may only be amended by unanimous consent of all Parties in writing.

### 7.6. **Assignment**

None of the Parties may assign or procure the assumption of its rights and obligations under this Agreement, either in whole or in part, to any other person without the prior written consent of the other Parties.

### 7.7. **Entire Agreement**

This Agreement constitutes the entire agreement between the Parties with respect to the subject matter of this Agreement. This Agreement supersedes any and all earlier agreements, either verbally or in writing, between the Parties in relation to the subject matter of this Agreement.

7.8. **Partial Invalidity**

The invalidity or unenforceability of any provision of this Agreement shall not affect the validity or enforceability of any other provision of this Agreement. Any such invalid or unenforceable provision shall be replaced or be deemed to be replaced by a provision that is considered to be valid and enforceable. The interpretation of the replacing provisions shall be as close as possible to the intent of the invalid or unenforceable provision.

7.9. **Compensation of costs**

Each of the Parties hereto shall pay its own expenses incurred or to be incurred in connection with this Agreement and matters incidental to this Agreement, except for the expenses incurred or to be incurred by the Trust Foundation, the Existing Investors and the New Investor I, which expenses shall be borne by the Company.

7.10. **No Waiver**

No failure by any Party to exercise, and no delay in exercising, any right under this Agreement, in the event of breach of contract by any Party hereto, will operate as a waiver of such right or any other right under this Agreement.

7.11. **No Rescission**

The Parties waive their right to rescind (*ontbinden*) this Agreement pursuant to article 2:265 DCC after Completion.

7.12. **Counterparts**

This Agreement may be signed in any number of counterparts each of which, when executed by one or more of the Parties, shall constitute an original.

7.13. **Governing Law**

This Agreement is governed by the laws of the Netherlands.

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7.14. **Jurisdiction**

All disputes arising in connection with this Agreement shall be finally settled in accordance with the rules of the Netherlands Arbitration Institute (*Nederlands Arbitrage Instituut*) and:

- 7.14.1. the arbitral tribunal shall be composed of three arbitrators;
- 7.14.2. the place of arbitration will be Amsterdam, the Netherlands;
- 7.14.3. the language of the proceedings will be English (unless the Parties agree otherwise);
- 7.14.4. the arbitrators will decide according to the rules of Dutch law;
- 7.14.5. the arbitral award will be final and binding;
- 7.14.6. to ensure that the arbitral award shall not be published, each Party shall notify the administrator of the NAI within one calendar month after receipt of the arbitral award that they object to publication of the arbitral award by the NAI;
- 7.14.7. the proceedings shall not be consolidated with other arbitral proceedings pursuant to Article 1046 of the Dutch Code of Civil Procedure.

*[signature pages to follow]*

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**This Agreement has been entered into on the date first above written.**

**Coöperatieve AAC LS U.A.**

/s/ Signature Illegible

By: Forbion 1 Management B.V.

Title: Director

By: Name Illegible

Title: Partner

**Forbion Co-Investment Coöperatief U.A.**

/s/ Signature Illegible

By: Forbion 1 Management B.V.

**Coöperatieve AAC LS U.A.**

/s/ Signature Illegible

By: Forbion 1 Management B.V.

Title: Director

By: Name Illegible

Title: Partner

**Forbion Co-Investment Coöperatief U.A.**

/s/ Signature Illegible

By: Forbion 1 Management B.V.

Title: Director  
By: Name Illegible  
Title: Partner

**Forbion Co-Investment II Coöperatief U.A.**

/s/ Signature Illegible  
By: Forbion 1 Co II Management B.V.

Title: Director  
By: Name Illegible  
Title: Partner

**Coöperatieve Gilde Healthcare II U.A.**

/s/ Edwin de Gaaf  
By: Gilde Healthcare II Management B.V.

Title: Director  
By: Edwin de Gaaf  
Title: \_\_\_\_\_

**Stichting Administratiekantoor uniQure B.V.**

/s/ Signature Illegible  
By: Forbion 1 Co II Management B.V.

Title: Director  
By: Name Illegible  
Title: \_\_\_\_\_

**uniQure B.V.**

/s/ Aldag  
By: \_\_\_\_\_  
Title: Director

Title: Director  
By: Name Illegible  
Title: Partner

**Forbion Co-Investment II Coöperatief U.A.**

/s/ Signature Illegible  
By: Forbion 1 Co II Management B.V.

Title: Director  
By: Name Illegible  
Title: Partner

**Stichting Administratiekantoor uniQure B.V.**

/s/ Signature Illegible  
By: Forbion 1 Co II Management B.V.

Title: Director  
By: Name Illegible  
Title: \_\_\_\_\_

**uniQure B.V.**

/s/ P. J. Morgan  
By: P. J. Morgan  
Title: Director

**SCHEDULE A (PARTIES)**

- (1) **Coöperatieve AAC LS U.A.**, a coöperative (*coöperatie*) incorporated under the laws of the Netherlands with its corporate seat in Amsterdam, and its business address at Gooimeer 2 35, 1411 DC Naarden, the Netherlands and registered with the Commercial Register of Gooi-, Eem- en Flevoland under number 34256402, represented by its managing director **Forbion 1 Management B.V.**, a private company with limited liability (*besloten vennootschap met beperkte aansprakelijkheid*), incorporated under the laws of the Netherlands with its registered office (*zetel*) in Amsterdam, and its business address at Gooimeer 2 35, 1411 DC Naarden, the Netherlands and registered with the Commercial Register of Gooi-, Eem- en Flevoland under number 34249898);
- (2) **Forbion Co-Investment Coöperatief U.A.**, a coöperative (*coöperatie*) incorporated under the laws of the Netherlands with its registered office in Naarden, and its business address at Gooimeer 2 35, 1411 DC Naarden, the Netherlands and registered with the Commercial Register of Gooi-, Eem- en Flevoland under number 32142360, represented by its managing director **Forbion 1 Management B.V.**, a private company with limited liability (*besloten vennootschap met beperkte aansprakelijkheid*), incorporated under the laws of the Netherlands with its registered office (*zetel*) in Amsterdam, and its business address at Gooimeer 2 35, 1411 DC Naarden, the Netherlands and registered with the Commercial Register of Gooi-, Eem- en Flevoland under number 34249898);
- (3) **Forbion Co-Investment II Coöperatief U.A.**, a coöperative (*coöperatie*) incorporated under the laws of the Netherlands with its registered office in Naarden, and its business address at Gooimeer 2 35, 1411 DC Naarden, the Netherlands and registered with the Commercial Register of Gooi-, Eem- en Flevoland under number 53958713, represented by its managing director **Forbion 1 CO II Management B.V.**, a private company with limited liability (*besloten vennootschap met beperkte aansprakelijkheid*), incorporated under the laws of the Netherlands with its registered office (*zetel*) in Amsterdam, and its business address at Gooimeer 2 35, 1411 DC Naarden, the Netherlands and registered with the Commercial Register of Gooi-, Eem- en Flevoland under number 53951956);
- (4) **Coöperatieve Gilde Healthcare II U.A.**, a coöperative (*coöperatie*) incorporated under the laws of the Netherlands with its corporate seat in Utrecht, and its business address at Newtonlaan 91 (3584 BP), the Netherlands and registered with the Commercial Register of Midden-Nederland under number 30216414, represented by its managing director **Gilde Healthcare II Management B.V.**, a private company with limited liability (*besloten vennootschap met beperkte aansprakelijkheid*), incorporated under the laws of the Netherlands with its registered office (*zetel*) seat in Utrecht, and its business address at Newtonlaan 91 (3584 BP), the Netherlands and registered with the Commercial Register of Midden-Nederland under number 30215056;
- (5) **Sichting Administratiekantoor uniQure B.V.**, a foundation organised under the laws of the Netherlands, having its registered office in Amsterdam, at Meibergdreef 61, 1105 BA Amsterdam Zuidoost, the Netherlands and registered with the Commercial Register of Amsterdam under number 55055036, duly represented by **Forbion 1 Co II Management B.V.**, **Forbion 1 CO II Management B.V.**, a private company with limited liability (*besloten vennootschap met beperkte aansprakelijkheid*), incorporated under the laws of the Netherlands with its registered office (*zetel*) in Amsterdam, and its business address at Gooimeer 2 35, 1411 DC Naarden, the Netherlands and registered with the Commercial Register of Gooi-, Eem- en Flevoland under number 53951956);

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- (6) **uniQure B.V.**, a private company with limited liability (*besloten vennootschap met beperkte aansprakelijkheid*), incorporated under the laws of the Netherlands with its registered office in Amsterdam, and its business address at Meibergdreef 61, 1105 BA Amsterdam Zuidoost, the Netherlands and registered with the Commercial Register of Amsterdam under number 54385229, duly represented by its managing directors Mr. J. Aldag and Mr. P. Morgan.

## SCHEDULE B (ACCESSION AGREEMENT)

This Accession Agreement (the “**Agreement**”) is made on [·]

### RECITALS:

- (A) [Note: insert name] (the “**New Shareholder**”) on [Note: insert date] acquired [Note: insert number] Ordinary Shares Class A by [an issue of new Shares] [a transfer of Shares by [Note: insert name of transferor] the “**Transferor**”)].
- (B) This Agreement is entered into in compliance with the terms of the class B shareholders agreement dated [Note: insert date] between the Existing Investors, the New Investors, the Company and the Trust Foundation (all as defined therein) (which agreement is referred to in this Agreement as the “**Class B Shareholders Agreement**”).

**IT IS AGREED** as follows:

- (1) Definitions used in this Agreement have the same meaning as given to them in the Class B Shareholders Agreement unless stated otherwise and the provisions of Clause 1 (Interpretation) of the Class B Shareholders Agreement shall apply to this Agreement.
- (2) The New Shareholder agrees to become a Party to the Class B Shareholders Agreement and to be bound by the terms of the Class B Shareholders Agreement in all respects as a Shareholder. [The New Shareholder assumes all the obligations of the Transferor in that capacity under the Class B Shareholders Agreement.]
- (3) SCHEDULE A (Parties) to the Class B Shareholders Agreement shall be updated to properly reflect the relevant changes in the ownership of the Shares.
- (4) The contact details of the new Shareholder are as set out in the updated version of SCHEDULE A (Parties), to be attached to the Class B Shareholders Agreement in replacement of the existing version.
- (5) [Note: insert other relevant details]
- (6) The provisions of the Clauses 7.4, 7.5, 7.6, 7.7, 7.8, 7.12, 7.13 and 7.14 shall apply *mutatis mutandis* to this Agreement.

**THUS AGREED AND SIGNED ON** [·],

[Note: to be signed by New Shareholder and Parties to the Class B Shareholders Agreement]

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## ACCESSION AGREEMENT CLASS B SHAREHOLDERS AGREEMENT

This Accession Agreement (the “**Agreement**”) is made on 25 May 2012 and made between

- (1) **COÖPERATIEVE AAC LS U.A.**, a coöperative (*coöperatie*) incorporated under the laws of the Netherlands with its corporate seat in Amsterdam, and its business address at Gooimeer 2 35, 1411 DC Naarden, the Netherlands and registered with the Commercial Register of Gooi-, Eem- en Flevoland under number 34256402 (the “**Existing Investor I**”);
  - (2) **FORBION CO-INVESTMENT COÖPERATIEF U.A.**, a coöperative (*coöperatie*) incorporated under the laws of the Netherlands with its registered office in Naarden, and its business address at Gooimeer 2 35, 1411 DC Naarden, the Netherlands and registered with the Commercial Register of Gooi-, Eem- en Flevoland under number 32142360 (the “**Existing Investor II**”);
  - (3) **FORBION CO-INVESTMENT II COÖPERATIEF U.A.**, a coöperative (*coöperatie*) incorporated under the laws of the Netherlands with its registered office in Naarden, and its business address at Gooimeer 2 35, 1411 DC Naarden, the Netherlands and registered with the Commercial Register of Gooi-, Eem- en Flevoland under number 53958713 (the “**New Investor I**”);
  - (4) **COÖPERATIEVE GILDE HEALTHCARE II U.A.**, a coöperative (*coöperatie*) incorporated under the laws of the Netherlands with its corporate seat in Utrecht, and its business address at Newtonlaan 91 (3584 BP), the Netherlands and registered with the Commercial Register of Midden-Nederland under number 30216414 (the “**New Investor II**”);
  - (5) **STICHTING ADMINISTRATIEKANTOOR UNIQUE B.V.**, a foundation organised under the laws of the Netherlands, having its registered office in Amsterdam, at Meibergdreef 61, 1105 BA Amsterdam Zuidoost, the Netherlands and registered with the Commercial Register of Amsterdam under number 55055036 (the “**Trust Foundation**”);
  - (6) **UNIQUE B.V.**, a private company with limited liability (*besloten vennootschap met beperkte aansprakelijkheid*), incorporated under the laws of the Netherlands with its registered office in Amsterdam, and its business address at Meibergdreef 61, 1105 BA Amsterdam Zuidoost, the Netherlands and registered with the Commercial Register of Amsterdam under number 54385229 (the “**Company**”);
  - (7) **S.J.H. VAN DEVENTER C.V.**, a Dutch limited partnership (*commanditaire vennootschap*), having its registered address at 1411 DC Naarden, Gooimeer 2 35, registered in the trade register under number 32158843 (the “**New Shareholder 1**”);
  - (8) **CREDIT LYONNAIS INNOVATION 6**, a French venture capital fund (*Fonds Commun de Placement dans l’Innovation*), having its business location at 100 Boulevard de Montparnasse, 75014 Paris, France (the “**New Shareholder 2**”), which is represented by its managing company the French limited liability company (*société anonyme à directoire et conseil de surveillance*) OMNES Capital, incorporated and existing under the laws of France, having its registered address at 100 Boulevard du Montparnasse, 75014 Paris, France and registered with the French Registry of Commerce and Companies under number 428 711 196 TCS Paris (“**OMNES Capital**”);
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- (9) **LCL INNOVATION 1**, a French venture capital fund (*Fonds commun de placement dans l’innovation*), having its business location at 100 Boulevard de Montparnasse, 75014 Paris, France (the “**New Shareholder 3**”), which is represented by OMNES Capital;
  - (10) **CLVC**, a French corporation (*Société anonyme*), incorporated and existing under the laws of France, having its registered office at 100 Boulevard du Montparnasse, 75014 Paris, France, and registered with the French Registry of Commerce and Companies under number 434.465.514 RCS Paris, France (the “**New Shareholder 4**”), which is represented by OMNES Capital;
  - (11) **CLV 1**, a French *Fonds Commun de Placement à Risques*, having its business location at 100 Boulevard de Montparnasse, 75014 Paris, France (the “**New Shareholder 5**”), which is represented by OMNES Capital;
  - (12) **ADVENT PRIVATE EQUITY FUND IV**, an English partnership, incorporated and existing under the laws of the United Kingdom, having its registered address at 25 Buckingham Gate, London, SW1E 6LD, United Kingdom and registered in England and Wales under number LP10002 (the “**New Shareholder 6**”);
  - (13) **ADVENT MANAGEMENT IV LP**, a Scottish limited partnership, incorporated and existing under the laws of Scotland, having its registered address at 50 Lothian Road, Festival Square, Edinburgh, EH3 9WJ, United Kingdom and registered in Scotland under number SL005366 (the “**New Shareholder 7**”);
  - (14) **TIVERINA INVERSIONES S.L.**, a Spanish private company with limited liability, having its place of business at Calle Zurbano nº76 6ª, Madrid, Spain, with Tax Identification Number B-85381960 (the “**New Shareholder 8**”);
  - (15) **ABADAI INVERSIONES S.L.**, a Spanish private company with limited liability, having its place of business at Calle Zurbano nº76 6ª, Madrid, Spain, with Tax Identification Number B-85383511 (the “**New Shareholder 9**”);
  - (16) **SURRIC INVERSIONES S.L.**, a Spanish private company with limited liability, having its place of business at Calle Zurbano nº76 6ª, Madrid, Spain, with Tax Identification Number B-85381507 (the “**New Shareholder 10**”);
  - (17) **HILOS Y POLO S.L.**, a Spanish private company with limited liability, having its place of business at Calle Zurbano nº76 6ª, Madrid, Spain, with Tax Identification Number B-83914481 (the “**New Shareholder 11**”);
  - (18) **RAMON MORA-FIGUEROA MORA-FIGUEROA**, with professional domicile at Madrid, Calle Zurbano nº76 6ª and holder of a National Identity Card with number 31.241.827-F (the “**New Shareholder 12**”);

- (19) **ACTIVOS Y TENENCIAS 85 S.L.**, a Spanish private company with limited liability, having its place of business at Calle Ana Teresa 85 B, Madrid Spain, with Tax identification number B-84366111 (the “**New Shareholder 13**”);
- (20) **FUNDACION PARA EL DESARROLLO Y LA COOPERACION INTERNACIONAL**, having its place of business at Calle Jose Abascal nº44 2º, Madrid, Spain, with Tax identification number G-80787161 (the “**New Shareholder 14**”);

- (21) **FUNDACION UNIVERSITARIA DE NAVARRA**, having its place of business at Avd. Pio XII 53 1º, Pamplona, Spain, with Tax Identification Number G-31469125 (the “**New Shareholder 15**”);
- (22) **JOSE LUIS PASCUAL PLAZA**, with professional domicile at Madrid, Calle del Pastor 4 and holder of a National Identity Card with number 02.153.208-V (the “**New Shareholder 16**”);
- (23) **LUPUS ALPHA MICRO CHAMPIONS**, a Sub Fund of Lupus alpha Fonds, a Fonds Commun de placement under Luxembourg law, for which and on behalf wherof Lupus alpha Investment S.A., 69, route d’Esch, L-1470 Luxembourg, R.C.S. Luxembourg B-79272 (Management Company) acts (the “**New Shareholder 17**”); and
- (24) **LUPUS ALPHA ALL OPPORTUNITIES FUND**, a Sub Fund of Lupus alpha Fonds, a Fonds Commun de placement under Luxembourg law, for which and on behalf wherof Lupus alpha Investment S.A., 69, route d’Esch, L-1470 Luxembourg, R.C.S. Luxembourg B-79272 (Management Company) acts (the “**New Shareholder 18**”),

The parties to this Agreement are hereinafter collectively referred to as the “**Parties**” and individually as a “**Party**”. The New Shareholders 1 up to and including 18 collectively referred to as the “**New Shareholders**” and individually as a “**New Shareholder**”.

#### RECITALS:

- (A) By an issue of new Ordinary Shares Class A pursuant to conversion of Ordinary Shares Class B following the Exchange Offer:
- (i) The Existing Investor I will acquire 4,193,568 Ordinary Shares Class A;
  - (ii) The Existing Investor II will acquire 1,794,117 Ordinary Shares Class A;
  - (iii) The New Investor II will acquire 6,081,803 Ordinary Shares Class A;
  - (iv) The New Shareholder 1 will acquire 49,298 Ordinary Shares Class A;
  - (v) The New Shareholder 2 will acquire 576,520 Ordinary Shares Class A;
  - (vi) the New Shareholder 3 will acquire 512,462 Ordinary Shares Class A;
  - (vii) the New Shareholder 4 will acquire 401,320 Ordinary Shares Class A;
  - (viii) the New Shareholder 5 will acquire 69,716 Ordinary Shares Class A;
  - (ix) the New Shareholder 6 will acquire 3,724,371 Ordinary Shares Class A;
  - (x) the New Shareholder 7 will acquire 37,241 Ordinary Shares Class A;
  - (xi) the New Shareholder 8 will acquire 478,084 Ordinary Shares Class A;
  - (xii) the New Shareholder 9 will acquire 210,824 Ordinary Shares Class A;

- (xiii) the New Shareholder 10 will acquire 210,824 Ordinary Shares Class A;
- (xiv) the New Shareholder 11 will acquire 66,000 Ordinary Shares Class A;
- (xv) the New Shareholder 12 will acquire 48,000 Ordinary Shares Class A;
- (xvi) the New Shareholder 13 will acquire 31,764 Ordinary Shares Class A;
- (xvii) the New Shareholder 14 will acquire 269,768 Ordinary Shares Class A;
- (xviii) the New Shareholder 15 will acquire 230,631 Ordinary Shares Class A;
- (xix) the New Shareholder 16 will acquire 10,000 Ordinary Shares Class A;
- (xx) the New Shareholder 17 will acquire 1,560,000 Ordinary Shares Class A; and
- (xxi) the New Shareholder 18 will acquire 850,000 Ordinary Shares Class A.



(B) This Agreement is entered into in compliance with the terms of the class B shareholders agreement dated 19 April 2012 between the Existing Investors, the New Investors, the Trust Foundation and the Company (all as defined therein) (which agreement is referred to in this Agreement as the “**Class B Shareholders Agreement**”).

The New Shareholder 1 up to and including the New Shareholder 18 collectively referred to as the “**New Shareholders**” and individually as a “**New Shareholder**”.

**IT IS AGREED** as follows:

- (1) Definitions used in this Agreement have the same meaning as given to them in the Class B Shareholders Agreement unless stated otherwise and the provisions of Clause 1 (Interpretation) of the Class B Shareholders Agreement shall apply to this Agreement.
- (2) With effect from the date of this Agreement, the New Shareholder becomes a Party to and will be bound by the terms of the Class B Shareholders Agreement in all respects as an Investor, under the condition precedent (*opschortende voorwaarde*) that the New Shareholder has acquired the Ordinary Shares Class A pursuant to the execution of the Exchange Offer as referred to in Recital A above.
- (3) SCHEDULE A (Parties) to the Class B Shareholders Agreement shall be updated to properly reflect the relevant changes in the ownership of the Shares.
- (4) The provisions of the Clauses 7.4, 7.5, 7.6, 7.7, 7.8, 7.12, 7.13 and 7.14 of the Class B Shareholders Agreement shall apply *mutatis mutandis* to this Agreement.

**THUS AGREED AND SIGNED ON 25 May 2012**

[signature pages to follow]

**This Agreement has been entered into on the date first above written.**

**Coöperatieve AAC LS U.A.**

/s/ H. A. Slootweg  
By: Forbion 1 Management B.V.  
  
Title: Director  
By: H. A. Slootweg  
Title: Director

**Forbion Co-Investment Coöperatief U.A.**

/s/ H. A. Slootweg  
By: Forbion 1 Management B.V.  
  
Title: Director  
By: H. A. Slootweg  
Title: Director

**Forbion Co-Investment II Coöperatief U.A.**

/s/ H. A. Slootweg  
By: Forbion 1 Co II Management B.V.  
  
Title: Director  
By: H. A. Slootweg  
Title: Director

**Coöperatieve Gilde Healthcare II U.A.**

/s/ Edwin de Gaaf  
By: Gilde Healthcare II Management B.V.  
  
Title: Director  
By: Edwin de Gaaf  
Title: Managing Partner

**Stichting Administratiekantoor uniQure B.V.**

/s/ H. A. Slootweg  
By: Forbion 1 Co II Management B.V.  
  
Title: Director  
By: H. A. Slootweg

**Coöperatieve AAC LS U.A.**

/s/ M. A. van Osch  
By: Forbion 1 Management B.V.  
  
Title: Director  
By: M. A. van Osch  
Title: Director

**Forbion Co-Investment Coöperatief U.A.**

/s/ M. A. van Osch  
By: Forbion 1 Management B.V.  
  
Title: Director  
By: M. A. van Osch  
Title: Director

**Forbion Co-Investment II Coöperatief U.A.**

/s/ M. A. van Osch  
By: Forbion 1 Co II Management B.V.  
  
Title: Director  
By: M. A. van Osch  
Title: Director

**Stichting Administratiekantoor uniQure B.V.**

/s/ M. A. van Osch  
By: Forbion 1 Co II Management B.V.  
  
Title: Director  
By: M. A. van Osch

Title: Director

**uniQure B.V.**

/s/ Aldag  
By: ALDAG  
Title: Director

Title: Director

**uniQure B.V.**

/s/ P. J. Morgan  
By: P. J. Morgan  
Title: Director

**This Agreement has been entered into on the date first above written.**

**S.J.H. Van Deventer C.V.**

/s/ H. A. Slootweg  
By: Forbion Capital Partners Management Services B.V.

Title: General Partner  
By: H. A. Slootweg  
Title: Director

**S.J.H. Van Deventer C.V.**

/s/ M. A. van Osch  
By: Forbion Capital Partners Management Services B.V.

Title: General Partner  
By: M. A. van Osch  
Title: Director

**This Agreement has been entered into on the date first above written.**

**Credit Lyonnais Innovation 6**

/s/ Fabien Prévost

By: OMNES capital

Title: Managing company  
By: Fabien Prévost  
Title: Illegible

**LCL Innovation 1**

/s/ Fabien Prévost

By: OMNES capital

Title: Managing company  
By: Fabien Prévost  
Title: Illegible

**CLVC**

/s/ Fabien Prévost

By: Fabien Prévost  
Title: Illegible

**CLV 1**

/s/ Fabien Prévost

By: OMNES capital

Title: Managing company

By: Fabien Prévost  
Title: Illegible

**This Agreement has been entered into on the date first above written.**

Advent Private Equity Fund IV LP

/s/ Signature Illegible

By: Advent Management IV Limited Partnership acting as General Partner by  
Advent Venture Partners LLP acting as Manager

By: Name Illegible  
Title: Partner

Advent Management IV Limited Partnership

/s/ Signature Illegible

By: Advent Venture Partners LLP

By: Name Illegible  
Title: Partner

Advent Private Equity Fund IV LP

/s/ Signature Illegible

By: Advent Management IV Limited Partnership acting as General Partner by  
Advent Venture Partners LLP acting as Manager

By: Name Illegible  
Title: Partner

Advent Management IV Limited Partnership

/s/ Signature Illegible

By: Advent Venture Partners LLP

By: Name Illegible  
Title: Partner

This Agreement has been entered into on the date first above written.

Tiverina Inversiones S.L

/s/ Signature Illegible

By: Name Illegible

Title: Director

By:   
Title:

Tiverina Inversiones S.L

N/A

By:

Title: Director

By:   
Title:

Abadai Inversiones S.L

/s/ Signature Illegible

By: Name Illegible

Title: Director

By:   
Title:

Abadai Inversiones S.L

N/A

By:

Title: Director

By:   
Title:

Surric Inversiones S.L

/s/ Signature Illegible

By: Name Illegible

Title: Director

By:   
Title:

Surric Inversiones S.L

N/A

By:

Title: Director

By:   
Title:

Hilos y Polo S.L

/s/ Signature Illegible

By: Name Illegible

Title: Director

By:   
Title:

Hilos y Polo S.L

N/A

By:

Title: Director

By:   
Title:

This Agreement has been entered into on the date first above written.

Ramon Mora-Figueroa Mora-Figueroa

/s/ Signature Illegible

Activos y Tenencias 85 S.L

/s/ Signature Illegible

By: Name Illegible

Title: Director

By: \_\_\_\_\_  
Title: \_\_\_\_\_

Fundacion para el  
Desarrollo y la Cooperación Internacional

/s/ Signature Illegible

By: Name Illegible

Title: Director

By: \_\_\_\_\_  
Title: \_\_\_\_\_

Fundacion Universitaria de Navarra

/s/ Signature Illegible

By:

Title: Director

By: Name Illegible  
Title: Director

Jose Luis Pascual Plaza

/s/ Signature Illegible

Acitvos y Tenencias 85 S.L

N/A

By:

Title: Director

By: N/A  
Title: \_\_\_\_\_

Fundacion para el  
Desarrollo y la Cooperación Internacional

/s/ Signature Illegible

By: Name Illegible

Title: Director

By: \_\_\_\_\_  
Title: \_\_\_\_\_

Fundacion Universitaria de Navarra

/s/ Signature Illegible

By:

Title: Director

By: Name Illegible  
Title: CEO

This Agreement has been entered into on the date first above written.

Lupus Alpha Micro Champions

/s/ Signature Illegible /s/ Signature Illegible

By: Lupus alpha Investment S.A.

Title: Management Company  
By: Name Illegible Name Illegible  
Title: MD MD

Lupus Alpha All Opportunities Fund

/s/ Signature Illegible /s/ Signature Illegible

By: Lupus alpha Investment S.A.

Title: Management Company  
By: Name Illegible Name Illegible  
Title: MD MD

Cooperatieve AAC LS U.A.  
 Forbion Co-Investment Coöperatief U.A.  
 Forbion Co-Investment Ii Coöperatief U.A.  
 Coöperatieve Gilde Healthcare II U.A.  
 S.J.H. VAN DEVENTER C.V.

CREDIT Lyonnais Innovation 6  
 LCL Innovation 1  
 CLVC  
 CLV1

ADVENT PRIVATE EQUITY FUND IV  
 ADVENT MANAGEMENT IV LP

TIVERINA INVERSIONES S.L.  
 ABADAI INVERSIONES S.L.  
 SURRIC INVERSIONES S.L.  
 HILOS Y POLO S.L.,  
 RAMON MOR  
 A-FIGUEROA MORA-FIGUEROA  
 ACTIVOS Y TENENCIAS 85 S.L.  
 FUNDACION PARA EL  
 DESARROLLO Y LA COOPERACION INTERNACIONAL  
 FUNDACION UNIVERSITARIA DE NAVARRA

JOSE LUIS PASCUAL PLAZA  
 LUPUS ALPHA MICRO CHAMPIONSs  
 LUPUS ALPHA ALL OPPERTUNITIES FUND

AND

STICHTING ADMINISTRATIEKANTOOR uniQure B.V.

AND

CHIESI FARMACEUTICI S.p.A.

AND

UNIQUE B.V.

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## CLASS C SHAREHOLDERS AGREEMENT

relating to uniQure B.V.

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## SCHEDULE A (PARTIES)

## SCHEDULE B (ACCESSION AGREEMENT)

THIS CLASS C SHAREHOLDERS AGREEMENT (the “Agreement”) is made on July 8, 2013.

### BETWEEN:

- (1) **Coöperatieve AAC LS U.A.**, a coöperative (*coöperatie*) incorporated under the laws of the Netherlands with its corporate seat in Amsterdam, and its business address at Gooimeer 2 35, 1411 DC Naarden, the Netherlands and registered with the Commercial Register of Gooi-, Eem- en Flevoland under number 34256402 (the “Existing Investor I”);
- (2) **Forbion Co-Investment Coöperatief U.A.**, a coöperative (*coöperatie*) incorporated under the laws of the Netherlands with its registered office in Naarden, and its business address at Gooimeer 2 35, 1411 DC Naarden, the Netherlands and registered with the Commercial Register of Gooi-, Eem- en Flevoland under number 32142360 (the “Existing Investor II”);
- (3) **Forbion Co-Investment II Coöperatief U.A.**, a coöperative (*coöperatie*) incorporated under the laws of the Netherlands with its registered office in Naarden, and its business address at Gooimeer 2 35, 1411 DC Naarden, the Netherlands and registered with the Commercial Register of Gooi-, Eem- en Flevoland under number 53958713 (the “Existing Investor III”);
- (4) **Coöperatieve Gilde Healthcare II U.A.**, a coöperative (*coöperatie*) incorporated under the laws of the Netherlands with its corporate seat in Utrecht, and its business address at Newtonlaan 91 (3584 BP), the Netherlands and registered with the Commercial Register of Midden-Nederland under number 30216414 (the “Existing Investor IV”);
- (5) **S.J.H. VAN DEVENTER C.V.**, a Dutch limited partnership (*commanditaire vennootschap*), having its registered address at 1411 DC Naarden, Gooimeer 2 35, registered in the trade register under number 32158843 (the “Existing Investor V”);
- (6) **CREDIT LYONNAIS INNOVATION 6**, a French venture capital fund (*Fonds Commun de Placement dans l’Innovation*), represented herein by its management company (*société de gestion*), Omnes Capital (formerly named Crédit Agricole Private Equity), a French corporation (*société par actions simplifiée*) with a share capital of 8,000,000 euros, the registered office of which is located at 37-41 rue du Rocher — 75008 Paris, France, registered with the French Registry of Commerce and Companies under number 428 711 196 RCS Paris (the “Existing Investor VI”);
- (7) **LCL INNOVATION 1**, a French venture capital fund (*Fonds Commun de Placement dans l’Innovation*), represented herein by its management company (*société de gestion*), Omnes Capital (formerly named Crédit Agricole Private Equity), a French corporation (*société par actions simplifiée*) with a share capital of 8,000,000 euros, the registered office of which is located at 37-41 rue du Rocher — 75008 Paris, France, registered with the French Registry of Commerce and Companies under number 428 711 196 RCS Paris (the “Existing Investor VII”);
- (8) **CLVC** (formerly named Crédit Lyonnais Venture Capital), a French corporation (*Société anonyme*) with a share capital of 14,786,948 euros, with its registered office located at 37-41 rue du Rocher — 75008 Paris, registered with the French Registry of Commerce and

Companies under number 434 465 514 RCS Paris, represented herein by Omnes Capital (formerly named Crédit Agricole Private Equity), a French Corporation (*société par actions simplifiée*) with a share capital of 8,000,000 euros, the registered office of which is located at 37-41 rue du Rocher — 75008 Paris, France, registered with the French Registry of Commerce and Companies under number 428 711 196 RCS Paris (the “Existing Investor VIII”);

- (9) **CLV1** (formerly named Crédit Lyonnais Venture 1), a French venture capital fund (*Fonds Commun de Placement à Risques*), represented herein by its managing partner, Omnes Capital (formerly named Crédit Agricole Private Equity), a French corporation (*société par actions simplifiée*) with a share capital of 8,000,000 euros, the registered office of which is located at 37-41 rue du Rocher — 75008 Paris, France, registered with the French Registry of Commerce and Companies under number 428 711 196 RCS Paris (the “Existing Investor IX”);
- (10) **ADVENT PRIVATE EQUITY FUND IV**, an English partnership, incorporated and existing under the laws of the United Kingdom, having its registered address at 25 Buckingham Gate, London, SW1E 6LD, United Kingdom and registered in England and Wales under number LP10002 (the “Existing Investor X”);

- (11) **ADVENT MANAGEMENT IV LP**, a Scottish limited partnership, incorporated and existing under the laws of Scotland, having its registered address at 50 Lothian Road, Festival Square, Edinburgh, EH3 9WJ, United Kingdom and registered in Scotland under number SL005366 (the “**Existing Investor XI**”);
- (12) **TIVERINA INVERSIONES S.L.**, a Spanish private company with limited liability, having its place of business at Calle Zurbano nº76 6ª, Madrid, Spain, with Tax Identification Number B-85381960 (the “**Existing Investor XII**”);
- (13) **ABADAI INVERSIONES S.L.**, a Spanish private company with limited liability, having its place of business at Calle Zurbano nº76 6ª, Madrid, Spain, with Tax Identification Number B-85383511 (the “**Existing Investor XIII**”);
- (14) **SURRIC INVERSIONES S.L.**, a Spanish private company with limited liability, having its place of business at Calle Zurbano nº76 6ª, Madrid, Spain, with Tax Identification Number B-85381507 (the “**Existing Investor XIV**”);
- (15) **HILOS Y POLO S.L.**, a Spanish private company with limited liability, having its place of business at Calle Zurbano nº76 6ª, Madrid, Spain, with Tax Identification Number B-83914481 (the “**Existing Investor XV**”);
- (16) **RAMON MORA-FIGUEROA MORA-FIGUEROA**, with professional domicile at Madrid, Calle Zurbano nº76 6ª and holder of a National Identity Card with number 31.241.827-F (the “**Existing Investor XVI**”);
- (17) **ACTIVOS Y TENENCIAS 85 S.L.**, a Spanish private company with limited liability, having its place of business at Calle Ana Teresa 85 B, Madrid Spain, with Tax identification number B-84366111 (the “**Existing Investor XVII**”);

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- (18) **FUNDACION PARA EL DESARROLLO Y LA COOPERACION INTERNACIONAL**, having its place of business at Calle Jose Abascal nº44 2º, Madrid, Spain, with Tax identification number G-80787161 (the “**Existing Investor XVIII**”);
- (19) **FUNDACION UNIVERSITARIA DE NAVARRA**, having its place of business at Avd. Pio XII 53 1º, Pamplona, Spain, with Tax Identification Number G-31469125 (the “**Existing Investor XIX**”);
- (20) **JOSE LUIS PASCUAL PLAZA**, with professional domicile at Madrid, Calle del Pastor 4 and holder of a National Identity Card with number 02.153.208-V (the “**Existing Investor XX**”);
- (21) **LUPUS ALPHA MICRO CHAMPIONS**, a Sub Fund of Lupus alpha Fonds, a Fonds Commun de placement under Luxembourg law, for which and on behalf whereof Lupus alpha Investment S.A., 69, route d’Esch, L-1470 Luxembourg, R.C.S. Luxembourg B-79272 (Management Company) acts (the “**Existing Investor XXI**”);
- (22) **LUPUS ALPHA ALL OPPORTUNITIES FUND**, a Sub Fund of Lupus alpha Fonds, a Fonds Commun de placement under Luxembourg law, for which and on behalf whereof Lupus alpha Investment S.A., 69, route d’Esch, L-1470 Luxembourg, R.C.S. Luxembourg B-79272 (Management Company) acts (the “**Existing Investor XXII**”);

and

- (23) **Stichting Administratiekantoor uniQure B.V.**, a foundation organised under the laws of the Netherlands, having its registered office in Amsterdam, at Meibergdreef 61, 1105 BA Amsterdam Zuidoost, the Netherlands and registered with the Commercial Register of Amsterdam under number 55055036 (the “**Trust Foundation**”);
- (24) **Chiesi Farmaceutici S.p.A** an Italian corporation, with its offices at Via Palermo, 26/A, 43122 Parma, Italy, (the “**New Investor**” or “**Chiesi**”)

and

- (25) **uniQure B.V.**, a private company with limited liability (*besloten vennootschap met beperkte aansprakelijkheid*), incorporated under the laws of the Netherlands with its registered office in Amsterdam, and its business address at Meibergdreef 61, 1105 BA Amsterdam Zuidoost, the Netherlands and registered with the Commercial Register of Amsterdam under number 54385229 (the “**Company**”);

The parties to this Agreement are hereinafter collectively referred to as the “**Parties**” and individually as a “**Party**”. Further details of the Parties are set out in SCHEDULE A (Parties). The Existing Investor I up to and including XXII and IV and the Trust Foundation are hereinafter jointly referred to as the “**Existing Investors**”. The Existing Investors and the New Investor, together with any other holder from time to time of Ordinary Shares Class A, Class B and Class C in the capital of the Company, are hereinafter collectively referred to as the “**Investors**” and individually as an “**Investor**”.

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#### RECITALS:

- (A) The New Investor and the Company have entered into that certain “**Subscription Agreement**” on 29 April 2013, pursuant to which the Company has agreed to issue Ordinary Shares Class C to Chiesi and Chiesi agreed to take new Class C Shares (the “**Transaction**”).
- (B) The Company is engaged in the development of human gene based therapies.
- (C) In this Agreement the Parties wish to set out the terms and conditions on which they have agreed to regulate the rights and obligations of the New Investor with respect to the ordinary shares Class C it holds from time to time.

IT IS AGREED as follows:

## 1. INTERPRETATION

### 1.1. In this Agreement, the following definitions are used:

“**Agreement**” means this shareholders agreement including schedules and appendices thereto as amended in accordance with its terms.

“**Articles of Association**” means the articles of association of the Company, as amended from time to time.

“**Asset Sale**” has the meaning given in Clause 4.2.

“**Business Day**” means a day (other than a Saturday or a Sunday) on which banks in the Netherlands are open for normal business.

“**Business Acquisition Agreement**” has the meaning given in the recitals of this Agreement.

“**Called Shareholders**” has the meaning given in Clause 3.3.1.

“**Called Shares**” has the meaning given in Clause 3.3.1.

“**Company**” has the meaning given in the opening of this Agreement.

“**DCC**” means the Dutch Civil Code.

“**Drag Along Notice**” has the meaning given in Clause 3.3.2.

“**Drag Along Option**” has the meaning given in Clause 3.3.1.

“**Existing Investor I up to and including XXII**” has the meaning given in the opening of this Agreement.

“**Existing Investors**” has the meaning given in the opening of this Agreement.

“**Exit**” has the meaning given in Clause 4.2.

“**Group**” means the Company together with its Subsidiaries.

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“**Investor**” and “**Investors**” has the meaning given in the opening of this Agreement.

“**Listing**” has the meaning given in Clause 4.2.

“**Liquidation**” means a liquidation, dissolution or winding-up (*liquidatie of ontbinding*) of the Company.

“**New Investor**” has the meaning given in the opening of this Agreement.

“**Ordinary Shares Class A**” means the ordinary shares class A (*gewone aandelen*) in the capital of the Company with a nominal value of EUR 0.01 each.

“**Ordinary Shares Class B**” means the ordinary shares class B (*gewone aandelen*) in the capital of the Company with a nominal value of EUR 0.01 each.

“**Ordinary Shares Class C**” means ordinary shares class C (*gewone aandelen*) in the capital of the Company with a nominal value of EUR 0.01 each.

“**Sale**” has the meaning given in Clause 3.2.1.

“**Selling Investor**” and “**Selling Investors**” has the meaning given in Clause 3.2.1.

“**Shareholder**” means any holder of Shares.

“**Shares**” means the issued and outstanding shares from time to time in the capital of the Company, consisting of Ordinary Shares Class A, Ordinary Shares Class B and Ordinary Shares Class C.

“**Subsidiary**” has the meaning given in article 2:24a of the DCC to the term “*dochtermaatschappij*”.

“**Tag Along Notice**” has the meaning given in Clause 3.2.1.

“**Tag Offer**” has the meaning given in Clause 3.2.3.

“**Third Party Purchaser**” has the meaning given in Clause 3.2.1.

“**Transaction**” has the meaning given in the recitals of this Agreement.

“**Trust Foundation**” has the meaning given in the opening of this Agreement.

### 1.2. In this Agreement, unless otherwise specified:

1.2.1. the masculine gender shall include the feminine and the neuter and vice versa;



1.2.2. references to a person shall include a reference to any individual, company, association, partnership, trust or joint venture (in each case whether or not having separate legal personality);

1.2.3. references to “include” and “including” shall be treated as references to “include without limitation” or “including without limitation”;

1.2.4. unless the context requires otherwise, words in the singular shall include the plural and vice versa;

1.2.5. the headings are for identification only and shall not affect the interpretation of this Agreement.

## 2. DIVIDEND POLICY

2.1.1. The Shareholders are entitled to the distribution of the profits of the Company for each Shareholder in proportion to the number of Shares that it holds.

2.1.2. The Company shall apply profits available for distribution for its further development and expansion prior to making any distributions to Shareholders.

## 3. TRANSFERS OF SHARES

### 3.1. General conditions Transfer

3.1.1. The New Investor shall not be authorised to transfer any of the Shares it holds, unless provided otherwise in this Agreement.

### 3.2. Tag Along

3.2.1. If an Investor or group of Investors holding 51% or more of the issued and outstanding Ordinary Shares Class A (the “**Selling Investor**” or “**Selling Investors**” as the case may be), that wishes (or wish) to sell any or all of the Shares held by it (or by them) to a person or any other person acting in concert with that person (a “**Third Party Purchaser**”) (a “**Sale**”), the Selling Investor(s) shall give not less than 15 Business Days advance notice of the proposed Sale to the Company and the New Investor (a “**Tag Along Notice**”).

3.2.2. The Tag Along Notice shall:

- (A) specify the number of Shares the Selling Investor(s) propose(s) to sell;
- (B) specify the name of the Third Party Purchaser;
- (C) specify the price (in cash or otherwise) per Share that the Third Party Purchaser is proposing to pay;
- (D) specify the proposed date of transfer;
- (E) be governed by the laws of the Netherlands;
- (F) specify the address where the counter notice should be sent.

3.2.3. As a condition to such Sale, the New Investor shall be entitled within 15 Business Days after receipt of the Tag Along Notice to notify the Selling Investor(s) that it wishes to sell a *pro rata parte* portion of the Shares held by it at the proposed price by sending a counter notice to the address specified in the Tag Along Notice (the “**Tag Offer**”).

### 3.3. Drag Along

3.3.1. In case a Selling Investor or Selling Investors, as the case may be, wish(es) to sell all, and for the avoidance of doubt, not less than all of the Shares held by it (or by them), to a Third Party Purchaser, the Selling Investor(s) shall have the right (the “**Drag Along Option**”), upon agreement on the terms and conditions of a bona fide offer by a Third Party Purchaser, to require all other Shareholders (the “**Called Shareholders**”) to sell and transfer all, and for the avoidance of doubt, no less than all, of their Shares (the “**Called Shares**”) to the proposed Third Party Purchaser in accordance with the provisions of this Clause 3.3.

3.3.2. The Selling Investor(s) that wish(es) to exercise the Drag Along Option shall give notice thereof in writing (the “**Drag Along Notice**”) to the Company and the Called Shareholders.

3.3.3. The transfer of the Called Shares to the Third Party Purchaser shall be completed within sixty (60) Business Days after service of the Drag Along Notice. The Called Shareholders shall be released from their obligation to transfer the Called Shares to the Third Party Purchaser if the Third Party Purchaser has not acquired the Called Shares on the expiration of such sixty (60) Business Day period.

3.3.4. If a Called Shareholder fails to cooperate with the transfer of its Called Shares to the Third Party Purchaser within a period of ten (10) Business Days from the proposed date of transfer, the Selling Investor(s) shall have the power and the duty to fulfil the obligations for and on behalf of the defaulting Called Shareholder.

3.3.5. Each Shareholder hereby grants an unconditional and irrevocable power of attorney to the Company to act on its behalf and to transfer the relevant Called Shares to the proposed purchaser in accordance with the provisions of this Clause 3.3.

3.3.6. The provisions of Clause 3.2 (Tag Along) do not apply to a (proposed) transfer of Shares of which a Drag Along Notice has been duly served pursuant to this Clause 3.3.

#### 4. ASSET SALE OR EXIT

4.1. It is the intention of the Parties that an Asset Sale or an Exit in particular a Listing (as defined in 4.2) be achieved as soon as practically possible and commercially sensible.

4.2. An Exit ("**Exit**") shall be:

- a transfer (or a series of related transfers) of all the Shares issued and outstanding;
- the listing and admission to trading on a market for listed securities of either (i) the Shares, (ii) an intermediate holding company's shares or (iii) the shares of new holding company established for the purposes of the Listing (a "**Listing**"); or
- a distribution pursuant to a winding-up or dissolution of the Company or any holding company of the Company, including following an Asset Sale.

4.3. An Asset Sale ("**Asset Sale**") shall be a sale by the Company and/or one or more of its Subsidiaries of all, or substantially all, of the Group's business, assets and undertaking.

4.4. A decision to achieve an Asset Sale requires the approval of the general meeting of shareholders of the Company and the affirmative vote of the Investors representing at least 51% of Ordinary Shares Class A.

4.5. Each Party (taking into account its rights and obligations under this Agreement) shall take all steps that are required to ensure the success of any proposed Exit or Asset Sale, subject always to fiduciary duties and compliance with applicable law, including that:

- (A) each Shareholder shall dispose of (a *pro rata parte* portion of) its Shares on the same terms and conditions as the other Shareholders; and
- (B) each of the Shareholders shall on a Listing, retain such number of Shares held at the time of the Listing for such period after the Listing as is required by the relevant listing rules or is recommended by the Company's financial advisors in such Listing.

#### 5. CONFIDENTIALITY

5.1. Subject to Clause 5.2, each Party shall treat as strictly confidential and not disclose or use any information relating to this Agreement or any ancillary matter and the negotiations leading up to this Agreement and including the disclosure or use of any information relating to the Group and its business operations, unless provided otherwise in this Agreement.

5.2. The restrictions contained in Clause 5.1 shall not apply if and to the extent:

- (A) disclosure is required by any law or by a court;
- (B) disclosure is required by any securities exchange or regulatory or governmental body;
- (C) disclosure is necessary to enforce this Agreement in court proceedings.
- (D) the other Parties have given their written consent to disclosure;
- (E) the information has come into the public domain through no fault of the relevant Party's group;
- (F) disclosure is necessary to obtain the advice of any professional adviser;
- (G) disclosure is necessary within the relevant Party's group.

In the event of a disclosure of information pursuant to Clause 5.2 (A) or (B), the disclosing Party shall consult with the other Parties (to the extent permitted by applicable laws or regulations) as to the contents, form and timing of the disclosure to be made.

5.3. The restrictions contained in this Clause 5.1 shall apply to each Party (as applicable) during the term of this Agreement and shall remain in full force and effect after a Shareholder ceases to be a Shareholder.

#### 6. TERM AND TERMINATION

##### 6.1. Term

This Agreement shall remain in full force and effect from the date of this Agreement until terminated in accordance with the terms of Clause 6.2.

##### 6.2. Termination

6.2.1. This Agreement can be terminated by unanimous consent of all Parties in writing.

6.2.2. This Agreement shall automatically terminate upon:

- (A) completion of an Exit or Asset Sale;
- (B) completion of a Liquidation;
- (C) acquisition by one Shareholder of all Shares;
- (D) disposal by the New Investor of all the Shares it holds without prejudice of article 11 of the Company's articles of association.

6.2.3. This Agreement shall terminate in respect of a Shareholder from the date it ceases to be a Shareholder.

## 6.3. **Surviving Clauses**

Termination of this Agreement shall be without prejudice to:

- 6.3.1. any right, liability or obligation accrued under this Agreement but not satisfied or discharged at the date of termination; and
- 6.3.2. the provisions of the Clauses 5 (Confidentiality), 7.3 (Notices), 7.12 (Governing Law) and 7.13 (Jurisdiction), which will remain in full force and effect.

## 7. **MISCELLANEOUS**

### 7.1. **Conflict**

Subject to applicable law, in case of an ambiguity or a conflict between provisions of the Articles of Association and provisions of this Agreement, the provisions of this Agreement shall prevail.

### 7.2. **Additional Investors**

- 7.2.1. No issue or transfer of Ordinary Shares Class C to any person who is not a Party to this Agreement shall be effectuated without first obtaining from such person a duly signed Accession Agreement in the form of SCHEDULE B (Accession Agreement), provided that the Parties to this Agreement will procure and will cooperate to sign such Accession Agreement in respect of any person that is

permitted to acquire Ordinary Shares Class C pursuant to this Agreement and to whom it is envisaged to transfer such Ordinary Shares Class C.

- 7.2.2. SCHEDULE A (Parties) shall be updated upon an issue or transfer of Shares to properly reflect the relevant changes.

### 7.3. **Notices**

All notices, consents, waivers and other communications under this Agreement must be in writing in Dutch or in English and delivered by hand or sent by registered mail, express courier, fax or e-mail to such addresses and fax numbers as a Party may notify to the other Parties from time to time. A notice shall be effective upon receipt and shall be deemed to have been received at the time of delivery, if delivered by hand, registered mail or express courier, or at the time of successful transmission, if delivered by fax or e-mail.

### 7.4. **Amendment**

This Agreement may only be amended by unanimous consent of all Parties in writing.

### 7.5. **Assignment**

None of the Parties may assign or procure the assumption of its rights and obligations under this Agreement, either in whole or in part, to any other person without the prior written consent of the other Parties.

### 7.6. **Entire Agreement**

This Agreement constitutes the entire agreement between the Parties with respect to the subject matter of this Agreement. This Agreement supersedes any and all earlier agreements, either verbally or in writing, between the Parties in relation to the subject matter of this Agreement.

### 7.7. **Partial Invalidity**

The invalidity or unenforceability of any provision of this Agreement shall not affect the validity or enforceability of any other provision of this Agreement. Any such invalid or unenforceable provision shall be replaced or be deemed to be replaced by a provision that is considered to be valid and enforceable. The interpretation of the replacing provisions shall be as close as possible to the intent of the invalid or unenforceable provision.

### 7.8. **Compensation of costs**

Each of the Parties hereto shall pay its own expenses incurred or to be incurred in connection with this Agreement and matters incidental to this Agreement, except for the expenses incurred or to be incurred by the Trust Foundation, the Existing Investors and the New Investor I, which expenses shall be borne by the Company.

### 7.9. **No Waiver**

7.10. **No Rescission**

The Parties waive their right to rescind (*ontbinden*) this Agreement pursuant to article 2:265 DCC after Completion.

7.11. **Counterparts**

This Agreement may be signed in any number of counterparts each of which, when executed by one or more of the Parties, shall constitute an original.

7.12. **Governing Law**

This Agreement is governed by the laws of the Netherlands.

7.13. **Jurisdiction**

All disputes arising in connection with this Agreement shall be finally settled in accordance with the rules of the Netherlands Arbitration Institute (*Nederlands Arbitrage Instituut*) and:

- 7.13.1. the arbitral tribunal shall be composed of three arbitrators;
- 7.13.2. the place of arbitration will be Amsterdam, the Netherlands;
- 7.13.3. the language of the proceedings will be English (unless the Parties agree otherwise);
- 7.13.4. the arbitrators will decide according to the rules of Dutch law;
- 7.13.5. the arbitral award will be final and binding;
- 7.13.6. to ensure that the arbitral award shall not be published, each Party shall notify the administrator of the NAI within one calendar month after receipt of the arbitral award that they object to publication of the arbitral award by the NAI;
- 7.13.7. the proceedings shall not be consolidated with other arbitral proceedings pursuant to Article 1046 of the Dutch Code of Civil Procedure.

[signature pages to follow]

This Agreement has been entered into on the date first above written.

**Coöperatieve AAC LS U.A.**

/s/H.A. Slootweg  
By: Forbion 1 Management B.V.  
  
Title: Director  
By: H.A. Slootweg  
Title: Director

**Coöperatieve AAC LS U.A.**

/s/M.A. van Osch  
By: Forbion 1 Management B.V.  
  
Title: Director  
By: M.A. van Osch  
Title: Director

**Forbion Co-Investment Coöperatief U.A.**

/s/H.A. Slootweg  
By: Forbion 1 Management B.V.  
  
Title: Director  
By: H.A. Slootweg  
Title: Director

**Forbion Co-Investment Coöperatief U.A.**

/s/M.A. van Osch  
By: Forbion 1 Management B.V.  
  
Title: Director  
By: M.A. van Osch  
Title: Director

**Forbion Co-Investment II Coöperatief U.A.**

/s/H.A. Slootweg  
By: Forbion 1 Co II Management B.V.  
  
Title: Director  
By: H.A. Slootweg  
Title: Director

**Forbion Co-Investment II Coöperatief U.A.**

/s/M.A. van Osch  
By: Forbion 1 Co II Management B.V.  
  
Title: Director  
By: M.A. van Osch  
Title: Director

/s/H.A. Slootweg  
By: S.J.H. van Deventer C.V.  
Forbion Capital Partners Management Services B.V. (text illegible)  
Title: Director  
By: H.A. Slootweg  
Title: Proxyholder

Coöperatieve Gilde Healthcare II U.A.

/s/Edwin de Graaf  
By: Gilde Healthcare II Management B.V.  
  
Title: Director  
By: Edwin de Graaf  
Title:

Coöperatieve Gilde Healthcare II U.A.

/s/ Pieter van der Meer  
By: Gilde Healthcare II Management B.V.  
  
Title: Director  
By: Pieter van der Meer  
Title:

Credit Lyonnais Innovation 6

By: Omnes Capital  
  
Title: Managing Company  
By:  
Title:

Credit Lyonnais Innovation 6

/s/Fabien Prévost  
By: Omnes Capital  
  
Title: Managing Company  
By: Fabien Prévost  
Title: CFO

CLVC

By:  
  
Title:  
By:  
Title:

CLVC

/s/Fabien Prévost  
By:  
  
Title:  
By: Fabien Prévost  
Title: CFO

LCL INNOVATION 1

By:  
  
Title: Managing Company  
By:  
Title:

LCL INNOVATION 1

/s/Fabien Prévost  
By:  
  
Title: Managing Company  
By: Fabien Prévost  
Title: CFO

CLV1

By:  
  
Title: Managing Company  
By:  
Title:

CLV1

/s/Fabien Prévost  
By:  
  
Title: Managing Company  
By: Fabien Prévost  
Title: CFO

LCL INNOVATION 1

By:  
  
Title:  
By:  
Title:

LCL INNOVATION 1

/s/Fabien Prévost  
By:  
  
Title:  
By: Fabien Prévost  
Title: CFO

ADVENT PRIVATE EQUITY FUND IV

/s/R.B. Parekh  
By: \_\_\_\_\_  
  
Title:  
By: R.B. Parekh  
Title: General Partner

Partner, Advent Venture Partners LLP  
Acting in its capacity as Manager of  
Advent Private Equity Fund IV

ADVENT MANAGEMENT IV LP

/s/R.B. Parekh  
By: \_\_\_\_\_  
  
Title:  
By: \_\_\_\_\_  
Title: General Partner

Partner, Advent Venture Partners LLP  
Acting in its capacity as Manager of  
Advent Management IV Limited Partnership

ADVENT PRIVATE EQUITY FUND IV

/s/Shahzad Malik  
By: \_\_\_\_\_  
  
Title:  
By: Shahzad Malik  
Title: General Partner

ADVENT MANAGEMENT IV LP

/s/Shahzad Malik  
By: \_\_\_\_\_  
  
Title:  
By: Shahzad Malik  
Title: General Partner

TIVERINA INVERSIONES S.L.

/s/Ramon Mora-Figueroa Mora-Figueroa  
By: \_\_\_\_\_  
  
Title:  
By: Ramon Mora-Figueroa Mora-Figueroa  
Title: Managing Director

ABADAI INVERSIONES S.L.

/s/Pablo Mora-Figueroa  
By: \_\_\_\_\_  
  
Title:  
By: Pablo Mora-Figueroa  
Title: Managing Director

SURRIC INVERSIONES S.L.

/s/Ramon Mora-Figueroa Mora-Figueroa  
By: \_\_\_\_\_  
  
Title:  
By: Ramon Mora-Figueroa Mora-Figueroa  
Title: Managing Director

HILOS Y POLO S.L.

/s/Ramon Mora-Figueroa Mora-Figueroa  
By: \_\_\_\_\_  
  
Title:  
By: Ramon Mora-Figueroa Mora-Figueroa  
Title: Managing Director

RAMON MORA-FIGUEROA MORA-FIGUEROA

/s/Ramon Mora-Figueroa Mora-Figueroa  
By: \_\_\_\_\_

TIVERINA INVERSIONES S.L.

By: \_\_\_\_\_  
  
Title:  
By: \_\_\_\_\_  
Title: \_\_\_\_\_

ABADAI INVERSIONES S.L.

By: \_\_\_\_\_  
  
Title:  
By: \_\_\_\_\_  
Title: \_\_\_\_\_

SURRIC INVERSIONES S.L.

By: \_\_\_\_\_  
  
Title:  
By: \_\_\_\_\_  
Title: \_\_\_\_\_

HILOS Y POLO S.L.

By: \_\_\_\_\_  
  
Title:  
By: \_\_\_\_\_  
Title: \_\_\_\_\_

RAMON MORA-FIGUEROA MORA-FIGUEROA

By: \_\_\_\_\_

Title: \_\_\_\_\_  
By: \_\_\_\_\_  
Title: \_\_\_\_\_

Title: \_\_\_\_\_  
By: \_\_\_\_\_  
Title: \_\_\_\_\_

ACTIVOS Y TENENCIAS 85 S.L.

[SIGNATURE ILLEGIBLE]  
By: \_\_\_\_\_  
  
Title: \_\_\_\_\_  
By: [NAME ILLEGIBLE]  
Title: Managing Director

ACTIVOS Y TENENCIAS 85 S.L.

By: \_\_\_\_\_  
  
Title: \_\_\_\_\_  
By: \_\_\_\_\_  
Title: \_\_\_\_\_

FUNDACION UNIVERSITARIA DE NAVARRA

[SIGNATURE ILLEGIBLE]  
By: \_\_\_\_\_  
  
Title: \_\_\_\_\_  
By: Director  
Title: [ILLEGIBLE]

FUNDACION UNIVERSITARIA DE NAVARRA

By: \_\_\_\_\_  
  
Title: \_\_\_\_\_  
By: \_\_\_\_\_  
Title: \_\_\_\_\_

JOSE LUIS PASCUAL PLAZA

[SIGNATURE ILLEGIBLE]  
By: \_\_\_\_\_  
  
Title: \_\_\_\_\_  
By: \_\_\_\_\_  
Title: \_\_\_\_\_

JOSE LUIS PASCUAL PLAZA

[SIGNATURE ILLEGIBLE]  
By: \_\_\_\_\_  
  
Title: \_\_\_\_\_  
By: \_\_\_\_\_  
Title: \_\_\_\_\_

FUNDACION PARA EL DESSARROLLO Y COOPERACION INTERNACIONAL

/s/Ramon Mora-Figueroa Mora-Figueroa  
By: \_\_\_\_\_  
  
Title: \_\_\_\_\_  
By: Ramon Mora-Figueroa Mora-Figueroa  
Title: Managing Director

FUNDACION PARA EL DESSARROLLO Y COOPERACION INTERNACIONAL

[SIGNATURE ILLEGIBLE]  
By: \_\_\_\_\_  
  
Title: \_\_\_\_\_  
By: [NAME ILLEGIBLE]  
Title: Managing Director

LUPUS ALPHA MICRO CHAMPIONS

/s/Michael Frick  
By: \_\_\_\_\_  
  
Title: Verwaltungsrat  
By: Michael Frick  
Title: \_\_\_\_\_

LUPUS ALPHA MICRO CHAMPIONS

/s/G. Albert  
By: \_\_\_\_\_  
  
Title: \_\_\_\_\_  
By: Göte Albert  
Title: Verwaltungsrat

LUPUS ALPHA ALL OPPERTUNITIES FUND

/s/Michael Frick  
By: \_\_\_\_\_  
  
Title: Verwaltungsrat  
By: Michael Frick  
Title: \_\_\_\_\_

LUPUS ALPHA ALL OPPERTUNITIES FUND

/s/G. Albert  
By: \_\_\_\_\_  
  
Title: \_\_\_\_\_  
By: Göte Albert  
Title: Verwaltungsrat

[SIGNATURE ILLEGIBLE]

By:

Title: Director

By: [NAME ILLEGIBLE]

Title: Director

[SIGNATURE ILLEGIBLE]

By:

Title: Director

By: [NAME ILLEGIBLE]

Title: Director

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**Chiesi Farmaceutici S.p.A.**

/s/Alberto Chiesi

By: Mr. Alberto Chiesi

Title: President

**Chiesi Farmaceutici S.p.A.**

/s/Ugo DiFrancesco

By: Mr. Ugo DiFrancesco

Title: CEO

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**uniQure B.V.**

/s/Aldag

By:

Title: CEO

By: Aldag

Title: CEO

**uniQure B.V.**

/s/PJ Morgan

By:

Title: CFO

By: PJ Morgan

Title: CFO

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#### SCHEDULE A (PARTIES)

- (1) **Coöperatieve AAC LS U.A.**, a coöperative (*coöperatie*) incorporated under the laws of the Netherlands with its corporate seat in Amsterdam, and its business address at Gooimeer 2 35, 1411 DC Naarden, the Netherlands and registered with the Commercial Register of Gooi-, Eem- en Flevoland under number 34256402, represented by its managing director **Forbion 1 Management B.V.**, a private company with limited liability (*besloten vennootschap met beperkte aansprakelijkheid*), incorporated under the laws of the Netherlands with its registered office (*zetel*) in Amsterdam, and its business address at Gooimeer 2 35, 1411 DC Naarden, the Netherlands and registered with the Commercial Register of Gooi-, Eem- en Flevoland under number 34249898);
- (2) **Forbion Co-Investment Coöperatief U.A.**, a coöperative (*coöperatie*) incorporated under the laws of the Netherlands with its registered office in Naarden, and its business address at Gooimeer 2 35, 1411 DC Naarden, the Netherlands and registered with the Commercial Register of Gooi-, Eem- en Flevoland under number 32142360, represented by its managing director **Forbion 1 Management B.V.**, a private company with limited liability (*besloten vennootschap met beperkte aansprakelijkheid*), incorporated under the laws of the Netherlands with its registered office (*zetel*) in Amsterdam, and its business address at Gooimeer 2 35, 1411 DC Naarden, the Netherlands and registered with the Commercial Register of Gooi-, Eem- en Flevoland under number 34249898);
- (3) **Forbion Co-Investment II Coöperatief U.A.**, a coöperative (*coöperatie*) incorporated under the laws of the Netherlands with its registered office in Naarden, and its business address at Gooimeer 2 35, 1411 DC Naarden, the Netherlands and registered with the Commercial Register of Gooi-, Eem- en Flevoland under number 53958713, represented by its managing director **Forbion 1 CO II Management B.V.**, a private company with limited liability (*besloten vennootschap met beperkte aansprakelijkheid*), incorporated under the laws of the Netherlands with its registered office (*zetel*) in Amsterdam, and its business address at Gooimeer 2 35, 1411 DC Naarden, the Netherlands and registered with the Commercial Register of Gooi-, Eem- en Flevoland under number 53951956);
- (4) **Coöperatieve Gilde Healthcare II U.A.**, a coöperative (*coöperatie*) incorporated under the laws of the Netherlands with its corporate seat in Utrecht, and its business address at Newtonlaan 91 (3584 BP), the Netherlands and registered with the Commercial Register of Midden-Nederland under number 30216414, represented by its managing director **Gilde Healthcare II Management B.V.**, a private company with limited liability (*besloten vennootschap met beperkte aansprakelijkheid*), incorporated under the laws of the Netherlands with its registered office (*zetel*) seat in Utrecht, and its business address at Newtonlaan 91 (3584 BP), the Netherlands and registered with the Commercial Register of Midden-Nederland under number 30215056
- (5) **S.J.H. VAN DEVENTER C.V.**, a Dutch limited partnership (*commanditaire vennootschap*), having its registered address at 1411 DC Naarden, Gooimeer 2 35, registered in the trade register under number 32158843
- (6) **CREDIT LYONNAIS INNOVATION 6**, a French venture capital fund (*Fonds Commun de Placement dans l'Innovation*), represented herein by its management company (*société de gestion*), Omnes Capital (formerly named Crédit Agricole Private Equity), a French corporation (*société par actions simplifiée*) with a share capital of 8,000,000 euros, the registered office of which is located at 37-41 rue du Rocher — 75008 Paris, France, registered with the French Registry of

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- (7) **LCL INNOVATION 1**, a French venture capital fund (*Fonds Commun de Placement dans l'Innovation*), represented herein by its management company (*société de gestion*), Omnes Capital (formerly named Crédit Agricole Private Equity), a French corporation (*société par actions simplifiée*) with a share capital of 8,000,000 euros, the registered office of which is located at 37-41 rue du Rocher — 75008 Paris, France, registered with the French Registry of Commerce and Companies under number 428 711 196 RCS Paris
- (8) **CLVC** (formerly named Crédit Lyonnais Venture Capital), a French corporation (*Société anonyme*) with a share capital of 14,786,948 euros, with its registered office located at 37-41 rue du Rocher — 75008 Paris, registered with the French Registry of Commerce and Companies under number 434 465 514 RCS Paris, represented herein by Omnes Capital (formerly named Crédit Agricole Private Equity), a French Corporation (*société par actions simplifiée*) with a share capital of 8,000,000 euros, the registered office of which is located at 37-41 rue du Rocher — 75008 Paris, France, registered with the French Registry of Commerce and Companies under number 428 711 196 RCS Paris
- (9) **CLV1** (formerly named Crédit Lyonnais Venture 1), a French venture capital fund (*Fonds Commun de Placement à Risques*), represented herein by its managing partner, Omnes Capital (formerly named Crédit Agricole Private Equity), a French corporation (*société par actions simplifiée*) with a share capital of 8,000,000 euros, the registered office of which is located at 37-41 rue du Rocher — 75008 Paris, France, registered with the French Registry of Commerce and Companies under number 428 711 196 RCS Paris
- (10) **ADVENT PRIVATE EQUITY FUND IV**, an English partnership, incorporated and existing under the laws of the United Kingdom, having its registered address at 25 Buckingham Gate, London, SW1E 6LD, United Kingdom and registered in England and Wales under number LP10002
- (11) **ADVENT MANAGEMENT IV LP**, a Scottish limited partnership, incorporated and existing under the laws of Scotland, having its registered address at 50 Lothian Road, Festival Square, Edinburgh, EH3 9WJ, United Kingdom and registered in Scotland under number SL005366
- (12) **TIVERINA INVERSIONES S.L.**, a Spanish private company with limited liability, having its place of business at Calle Zurbano nº76 6ª, Madrid, Spain, with Tax Identification Number B-85381960
- (13) **ABADAI INVERSIONES S.L.**, a Spanish private company with limited liability, having its place of business at Calle Zurbano nº76 6ª, Madrid, Spain, with Tax Identification Number B-85383511
- (14) **SURRIC INVERSIONES S.L.**, a Spanish private company with limited liability, having its place of business at Calle Zurbano nº76 6ª, Madrid, Spain, with Tax Identification Number B-85381507
- (15) **HILOS Y POLO S.L.**, a Spanish private company with limited liability, having its place of business at Calle Zurbano nº76 6ª, Madrid, Spain, with Tax

Identification Number B-83914481

- (16) **RAMON MORA-FIGUEROA MORA-FIGUEROA**, with professional domicile at Madrid, Calle Zurbano nº76 6ª and holder of a National Identity Card with number 31.241.827-F
- (17) **ACTIVOS Y TENENCIAS 85 S.L.**, a Spanish private company with limited liability, having its place of business at Calle Ana Teresa 85 B, Madrid Spain, with Tax identification number B-84366111
- (18) **FUNDACION PARA EL DESARROLLO Y LA COOPERACION INTERNACIONAL**, having its place of business at Calle Jose Abascal nº44 2º, Madrid, Spain, with Tax identification number G-80787161
- (19) **FUNDACION UNIVERSITARIA DE NAVARRA**, having its place of business at Avd. Pio XII 53 1º, Pamplona, Spain, with Tax Identification Number G-31469125
- (20) **JOSE LUIS PASCUAL PLAZA**, with professional domicile at Madrid, Calle del Pastor 4 and holder of a National Identity Card with number 02.153.208-V
- (21) **LUPUS ALPHA MICRO CHAMPIONS**, a Sub Fund of Lupus alpha Fonds, a Fonds Commun de placement under Luxembourg law, for which and on behalf wherof Lupus alpha Investment S.A., 69, route d'Esch, L-1470 Luxembourg, R.C.S. Luxembourg B-79272 (Management Company) acts
- (22) **LUPUS ALPHA ALL OPPORTUNITIES FUND**, a Sub Fund of Lupus alpha Fonds, a Fonds Commun de placement under Luxembourg law, for which and on behalf wherof Lupus alpha Investment S.A., 69, route d'Esch, L-1470 Luxembourg, R.C.S. Luxembourg B-79272 (Management Company) acts

And

- (23) **Sichting Administratiekantoor uniQure B.V.**, a foundation organised under the laws of the Netherlands, having its registered office in Amsterdam, at Meibergdreef 61, 1105 BA Amsterdam Zuidoost, the Netherlands and registered with the Commercial Register of Amsterdam under number 55055036, duly represented by its board members Mr. W. Stevens, W. Swarte and R. Jongejan, a private company with limited liability (*besloten vennootschap met beperkte aansprakelijkheid*), incorporated under the laws of the Netherlands with its registered office (*zetel*) in Amsterdam, and its business address at Gooimeer 2 35, 1411 DC Naarden, the Netherlands and registered with the Commercial Register of Gooi-, Eem- en Flevoland under number 53951956);

And

- (24) **uniQure B.V.**, a private company with limited liability (*besloten vennootschap met beperkte aansprakelijkheid*), incorporated under the laws of the Netherlands with its registered office in Amsterdam, and its business address at Meibergdreef 61, 1105 BA Amsterdam Zuidoost, the Netherlands and registered with the Commercial Register of Amsterdam under number 54385229, duly represented by its managing directors Mr. J. Aldag and Mr. P. Morgan

and

- (25) Chiesi Farmaceutici S.p.A. an Italian corporation, with its offices at Via Palermo, 26/A, 43122 Parma, Italy and registered with the Commercial Register of [ ] under number [ ], duly represented by [ ].

## SCHEDULE B (ACCESSION AGREEMENT)

This Accession Agreement (the “**Agreement**”) is made on [·]

### RECITALS:

- (A) [Note: insert name] (the “**New Shareholder**”) on [Note: insert date] acquired [Note: insert number] Ordinary Shares Class C by [an issue of new Shares] [a transfer of Shares by [Note: insert name of transferor] the “**Transferor**”)].
- (B) This Agreement is entered into in compliance with the terms of the Class C shareholders agreement dated [Note: insert date] between the Existing Investors, the New Investor, the Company and the Trust Foundation (all as defined therein) (which agreement is referred to in this Agreement as the “**Class C Shareholders Agreement**”).

**IT IS AGREED** as follows:

- (1) Definitions used in this Agreement have the same meaning as given to them in the Class C Shareholders Agreement unless stated otherwise and the provisions of Clause 1 (Interpretation) of the Class C Shareholders Agreement shall apply to this Agreement.
- (2) The New Shareholder agrees to become a Party to the Class C Shareholders Agreement and to be bound by the terms of the Class C Shareholders Agreement in all respects as a Shareholder. [The New Shareholder assumes all the obligations of the Transferor in that capacity under the Class C Shareholders Agreement.]
- (3) SCHEDULE A (Parties) to the Class C Shareholders Agreement shall be updated to properly reflect the relevant changes in the ownership of the Shares.
- (4) The contact details of the new Shareholder are as set out in the updated version of SCHEDULE A (Parties), to be attached to the Class C Shareholders Agreement in replacement of the existing version.
- (5) [Note: insert other relevant details]
- (6) The provisions of the Clauses 7.3, 7.4, 7.5, 7.6, 7.7, 7.11, 7.12 and 7.13 shall apply *mutatis mutandis* to this Agreement.

**THUS AGREED AND SIGNED ON** [·],

[Note: to be signed by New Shareholder and Parties to the Class C Shareholders Agreement]

Rutgers Posch Visée Endedijk N.V.  
 Herengracht 466, 1017 CA Amsterdam  
 P.O. Box 10896, 1001 EW Amsterdam  
 The Netherlands  
 T + 31(0)20 891 3900 | F + 31(0)20 891 3901  
 www.rutgersposch.com

## Draft

uniQure B.V. (the “Issuer”)  
 Meibergdreef 61  
 1105 BA Amsterdam  
 The Netherlands

Date: [ ] 2013

## uniQure B.V. - SEC Exhibit 5 opinion letter

Dear Sirs,

### 1. Introduction

We have acted as Dutch legal counsel to the Issuer in connection with the Registration (as defined below).

### 2. Definitions

Certain terms used in this opinion are defined in Annex 1 (*Definitions*) hereto.

### 3. Dutch Law

We express no opinion or statement on any law other than Dutch Law. Our investigation has further been limited to the text of documents. We have not investigated the meaning and effect of any document governed by a law other than Dutch Law. The opinions and statements expressed herein are rendered only as of the date of this opinion and we assume no obligation to advise you of facts, circumstances, events or changes in Dutch Law that may hereafter arise or be brought to our attention and that may alter, affect or modify the opinions or statements expressed herein.

### 4. Scope of investigation; definitions

For purposes of this opinion, we have examined and relied solely upon the following documents:

*Rutgers & Posch is the tradename of Rutgers Posch Visée Endedijk N.V. in Amsterdam (Traderegister no. 56919891). The general terms and conditions of Rutgers & Posch, which stipulate a limitation of liability, the applicability of Dutch law and the exclusive jurisdiction of the district court in Amsterdam, are applicable to all work performed. The general terms and conditions are available at [www.rutgersposch.com](http://www.rutgersposch.com).*

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- 4.1 a copy of the Registration Statement;
  - 4.2 a copy of the Deed of Incorporation;
  - 4.3 a copy of the Extract;
  - 4.4 a copy of the Shareholders Register;
  - 4.5 a copy of the IPO Resolution;
  - 4.6 [a copy/the form] of the Shareholders Resolution;
  - 4.7 a copy of the Board Certificate;
  - 4.8 the form of the Deed of Conversion containing the Issuer’s articles of association (*statuten*) as will be in force at the time of the Issue of the Registration Shares;
  - 4.9 the form of the Underwriting Agreement;
  - 4.10 [the form of each Pricing Resolution]; and
  - 4.11 the form of the Deed of Issuance.

In addition, we have examined such documents, and performed such other investigations, as we considered necessary for the purpose of this opinion.

### 5. Assumptions

For the purpose of this opinion, we have assumed that:

- 5.1 all copies of documents conform to the originals and that all originals are authentic and complete;

- 5.2 each signature is the genuine signature of the individual concerned;
- 5.3 all factual matters, statements in documents, confirmations and other results of our investigation, relied upon or assumed herein, were true and accurate on the date of signing of the Documents and remain true and accurate on the date hereof;
- 5.4 The Registration Statement has been or will have been filed with the SEC in the form referred to in this opinion;
- 5.5 The Deed of Conversion and Pricing Resolution will have been executed in the form

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referred to in this opinion; and

- 5.6 The Registration Shares will have been validly accepted by the subscriber for them.

## 6. Opinions

Based upon the foregoing and subject to any factual matters and documents not disclosed to us in the course of our investigation, and subject to the qualifications and limitations stated hereafter, we express the following opinion:

- 6.1 The Registration Shares, when issued, fully paid for and delivered in accordance with the terms of the Deed of Issuance will be validly issued, fully paid and non-assessable<sup>(1)</sup>.

## 7. Reliance

- 7.1 This opinion is furnished to you in order to be filed as an exhibit to the Registration Statement and may be relied upon for the purpose of the Registration. We consent to the filing of this opinion as Exhibit 5 to the Registration Statement and further consent to the reference to our firm in the Registration Statement under the caption “Legal Matters”. In giving such consent, we do not admit that we come within the category of persons whose consent is required under section 7 of the U.S. Securities Act of 1933 or the rules and regulations promulgated thereunder.
- 7.2 This opinion may only be relied upon under the express condition that it and any issues of interpretation or liability arising thereunder will be governed by Dutch Law and be brought before a court of the Netherlands. In addition, this opinion may only be relied upon under the express condition and limitation that any possible liability of Rutgers Posch Visée Endedijk N.V., its shareholders (including its directors) and employees is limited to the amount available and payable under Rutgers Posch Visée Endedijk N.V.’s professional malpractice insurance coverage.

Yours faithfully,

Rutgers Posch Visée Endedijk N.V.

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(1) The term “non-assessable” has no equivalent in Dutch and as used in this opinion it means that a holder of a share will not by reason of merely being such a holder, be subject to assessment or calls by the Issuer or its creditors for further payment (in addition to the amount required for the share to be fully paid) on such share.

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## Annex 1 Definitions

“**Board Certificate**” means the certificate dated the date of this opinion attached as Annex 2 hereto;

“**Chamber of Commerce**” means the Chamber of Commerce (*Kamer van Koophandel en Fabrieken*) of Amsterdam;

“**Deed of Conversion**” means the draft deed of conversion and amendment of the articles of association dated [ ] 2013 providing for the conversion of the Issuer from a private company with limited liability (*besloten vennootschap met beperkte aansprakelijkheid*) into a public limited liability company (*naamloze vennootschap*) and amendment of its articles of association;

“**Deed of Issuance**” means a copy of the [draft] private deed of issuance (*onderhandse akte van uitgifte*) in respect of the Registration Shares dated [ ] 2013;

“**Deed of Incorporation**” means the Company’s deed of incorporation dated 9 January 2013 as deposited with the Chamber of Commerce;

“**Dutch Law**” means the laws of the Kingdom of the Netherlands excluding Aruba, Bonaire, Curaçao, Saba, Sint Eustatius and Sint Maarten (“the Netherlands”) as they currently stand and are applied by the courts of the Netherlands but excluding unpublished case law and case law available in electronic form only;

“**Extract**” means the copy of a trade register extract regarding the Company obtained from the Chamber of Commerce and dated [ ] 2013;

“**IPO Resolution**” means the resolutions of the Issuer’s supervisory board set out in the [written resolutions dated /minutes of its meeting held on] [ ] 2013, including a resolution to appoint a pricing committee (the “**Pricing Committee**”) and delegate to the Pricing Committee the authority to (i) determine the number of and issue price for the Registration Shares, subject to certain conditions, and (ii) approve the terms (including discounts and commissions) of an underwriting agreement;

**“Insolvency Proceedings”** means each of the proceedings listed in Annex A or B of Council Regulation (EC) No 1346/2000 of 29 May 2000 on insolvency proceedings (OJ 2000, L 160, 1), as amended;

**“Issue Authorisation Maximum”** is defined in the definition of “Shareholders

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Resolution”;

**“Pricing Resolution”** means:

- (a) a draft written resolution of the Issuer’s management board dated [ ] 2013, including resolutions to:
  - (i) propose the number of and issue price for the Registration Shares;
  - (ii) propose to grant an option for a number of Registration Shares to be issued in addition to the number of Registration Shares referred to in (i);
  - (iii) propose to issue the Registration Shares; and
  - (iv) propose to exclude all pre-emption rights (*voorkeursrechten*) in respect of the issue of Registration Shares and the grant of the option to acquire Registration Shares;
- (b) a draft written resolution of the Pricing Committee dated [ ] 2013 to approve the resolution of the management board referred to in (a);
- (c) a draft written resolution of the Issuer’s [management board] dated [ ] 2013, to adopt the resolutions referred to in (a).
- (d) a draft written resolution of the Issuer’s supervisory board dated [ ] 2013 to approve the resolution of the management board referred to in (a); and
- (e) a draft written resolution of the Issuer’s management board dated [ ] 2013 to (i) determine the number of Registration Shares to be issued in addition to the number of Registration Shares referred to in paragraph (a)(ii) and (ii) issue such Registration Shares;

**“Registration”** means the registration of the Registration Shares with the SEC under the U.S. Securities Act;

**“Registration Shares”** means a maximum of [ ] ordinary shares, nominal value EUR [0.05] each, in the Issuer’s share capital, to be issued by the Issuer pursuant to the IPO Resolution, the Shareholders Resolution and the Pricing Resolution;

**“Registration Statement”** means the registration statement on form F-1 (Registration No. 333-188855) in relation to the Registration to be filed with the SEC on the date hereof (excluding any documents incorporated by reference in it and any exhibits to

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it);

**“SEC”** means the U.S. Securities and Exchange Commission;

**“Shareholders Register”** means the Issuer’s shareholders register;

**“Shareholders Resolution”** means the [written] resolutions of the Issuer’s general meeting of shareholders [set out in the extract from the minutes of its meeting held on / dated] [ ] 2013, including a resolution to designate the Issuer’s management board as the corporate body authorised to resolve, subject to the approval of the Issuer’s supervisory board, to issue ordinary shares in the Issuer’s capital up to a maximum of [ ] (the **“Issue Authorisation Maximum”**) and to exclude all pre-emption rights in respect thereof;

**“Underwriting Agreement”** means the draft underwriting agreement entered into between the Underwriters as representatives for the several underwriters named in Schedule A of the underwriting agreement dated [ ] 2013; and

**“U.S. Securities Act”** means the U.S. Securities Act of 1933, as amended.

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## Annex 2 Board Certificate

From: the management board of uniQure B.V.

Dated: [ ] 2013

The undersigned:

1. Jörn Aldag; and
2. Piers Morgan,

acting in their capacity as managing directors of uniQure B.V., a private limited liability company (*besloten vennootschap met beperkte aansprakelijkheid*) incorporated under Dutch law, having its corporate seat in Amsterdam, the Netherlands and registered with the Dutch trade register of the Chamber of Commerce under number 54385229 (the “**Issuer**”),

Background:

- I. The Issuer intends to seek the Registration with the SEC of the Registration Shares;
- II. In connection with the Registration, on the date of this Board Certificate, Rutgers Posch Visée Endedijk N.V. intends to issue a legal opinion in the form attached to this certificate (the “**Legal Opinion**”);
- III. This Board Certificate is the “Board Certificate” as defined in the Legal Opinion; and
- IV. The undersigned make the certifications in this Board Certificate after due and careful consideration and after having made all necessary enquiries.

#### 1. Construction

2.1 Terms defined in the Legal Opinion have the same meaning in this Board Certificate; and

2.2 In this Board Certificate “including” means “including without limitation”.

#### 2. Certification

Each undersigned certifies the following:

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#### 2.1 Authenticity

As at the date of this Board Certificate:

- i) all information regarding the Issuer registered or on file with the Dutch Trade Register of the Chamber of Commerce; and
- ii) all information in the Shareholders Register,

is correct, complete and up to date.

#### 2.2 Solvency

The Issuer is not the subject of any Insolvency Proceedings, emergency measure (*noodregeling*), a non-insolvency dissolution, or a statutory merger or demerger.

#### 2.3 Issue

The maximum number of Registration Shares to be issued will not exceed the Issue Authorisation Maximum.

#### 2.4 General

No undersigned is aware of:

- i) any claim (whether actual or threatened and including any claim, litigation, arbitration or administrative or regulatory proceedings) to the contrary of the certifications in this Board Certificate; or
- ii) any fact or circumstance which he or she understands or suspects has or might have any impact on the correctness of the Legal Opinion and which has not been disclosed to Rutgers Posch Visée Endedijk N.V. in writing.

#### 3. Reliance

Rutgers Posch Visée Endedijk N.V. may rely on this Board Certificate (without personal liability for the undersigned).

In evidence whereof this Board Certificate was signed in the manner set out below.

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Name: Jörn Aldag

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Name: Piers Morgan

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J.J. Viottastraat 52  
1071 JT Amsterdam  
The Netherlands

T +31 20 760 16 00  
info@vancampenliem.com  
www.vancampenliem.com

To:

uniQure B.V.  
Meibergdreef 61  
1105 BA Amsterdam  
The Netherlands

[\*\*\*], 2013

**Re: uniQure B.V. — SEC registration of common shares (EXHIBIT 8.2)**

Dear Sirs,

You have requested us to render an opinion on matters of Dutch law in relation to the registration of [•] ordinary shares (the “Shares” and each a “Share”) each with a nominal value of [•] in the capital of uniQure B.V. (the “Issuer”) with the SEC under the U.S. Securities and Exchange Commission (the “SEC”) as exhibit 8.2 to the registration statement (the “Registration Statement”) on Form F-1 (Registration No. [333-•]), in relation to the registration (the “Registration”) of the issuance and sale of the Shares.

### 1. Scope of Opinion

This opinion is given only with respect to Dutch law in force at the date of this opinion letter. It (including all terms used in it) is to be construed in accordance with Dutch law. No opinion is expressed or implied as to the laws of any other jurisdiction.

### 2. Documents Examined

For the purposes of rendering this opinion, we have examined copies of the following documents:

- a. the Registration Statement, as amended to date;
- b. the notarial deed of incorporation of the Issuer executed on 19 January 2012 (the “Incorporation Deed”);

ATTORNEYS AT LAW / CIVIL LAW NOTARIES / TAX ADVISORS

Van Campen Liem is the joint trade name of Liem & Partners N.V. and Van Campen & Partners N.V.  
Liem & Partners N.V. has its statutory seat at Amsterdam, the Netherlands, and is registered with the Trade Register under number 54787882.  
Van Campen & Partners N.V. has its statutory seat at Amsterdam, the Netherlands, and is registered with the Trade Register under number 54033500.

- c. the notarial deed of amendment of the articles of association of the Issuer, executed on July 24, 2013, which includes the articles of association of the Issuer as currently in force; and
- d. the notarial deed of amendment of the articles of association of the Issuer executed on [•] 2013 (the “Conversion Deed”).

### 3. Assumptions

For the purpose of rendering this opinion we have assumed:

- a. Each copy conforms to the original and each original is genuine and complete;
- b. The Deed of Conversion will have been executed in the form referred to in this opinion; and
- c. The Registration Statement has been or will have been filed with the SEC in the form referred to in this opinion.

### 4. Opinion

Based upon the foregoing (including the assumptions set forth above) and subject to the qualifications listed herein and subject to any facts, circumstances, events or documents not disclosed to us in the course of our examination referred to above, we are, at the date hereof, of the opinion that:

The statements in the Registration Statement under the heading “*Taxation in the Netherlands*” are a summary of the matters of Dutch tax law referred to therein, insofar as they include statements as to Dutch tax law (meaning any tax of whatever nature levied by or on behalf of the Netherlands or any of its subdivisions or taxing authorities), and that summary is true and accurate in all material respects.

### 5. Miscellaneous



This opinion expresses and describes Dutch legal concepts in English and not in their original Dutch terms; these concepts may not be identical to the concepts described by the English translations; this opinion may therefore be relied upon only on the express condition that it shall be governed by and that all words and expressions used herein shall be construed and interpreted in accordance with the laws of the Netherlands.

This opinion is an exhibit to the Registration Statement and may be relied upon only for the purpose of the Registration.

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This opinion is solely rendered by Liem & Partners N.V., with the exclusion of any of its officers, employees, legal professionals and affiliates, and Liem & Partners N.V. is the sole entity responsible for this opinion. Any liability of Liem & Partners N.V. pursuant to this opinion shall be limited to the amount covered by its liability insurance.

In issuing this opinion we do not assume any obligations to notify or to inform you of any developments subsequent to its date that might render its contents untrue or inaccurate in whole or in part of such time.

This opinion is strictly limited to the matters stated herein and may not read as extending by implication to any matters not specifically referred to. Nothing in this opinion should be taken as expressing an opinion in respect of any document examined in connection with this opinion except as expressly confirmed herein.

We hereby consent (the “**Consents**”) the Issuer to:

- a. file this opinion with the SEC as Exhibit 8.2 to the Registration Statement; and
- b. refer to Van Campen Liem (Liem & Partners N.V.) giving this opinion under the heading “Legal Matters” in the Registration Statement.

However, the Consents are not an admittance that we are in the category of persons whose consent is required under Section 7 of the Securities Act or any rules or regulations of the SEC promulgated thereunder.

Yours sincerely,

Van Campen Liem / Liem & Partners N.V.

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Confidential Materials omitted and filed separately with the Securities and Exchange Commission. Double asterisk denote omissions.

**PUBLIC HEALTH SERVICE**

**PATENT LICENSE AGREEMENT - NONEXCLUSIVE**

COVER PAGE

For PHS internal use only:

License Number:  
L - 1 0 7 - 2007 /0

License Application Number: A-274-2006

Serial Number(s) of Licensed Patent(s) or Patent Application(s):

I. U.S. Patent Application(s) or Patent(s):

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II. PCT or Foreign Patent Application(s) or Patent(s):

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Licensee: Amsterdam Molecular Therapeutics

Cooperative Research and Development Agreement (CRADA) Number (if a subject invention): N/A

Additional Remarks: None

A-274-2006

**CONFIDENTIAL**

PHS Patent License Agreement-Nonexclusive  
Model 10-2005 [Final] [AMT] [4-23-2007]

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Public Benefit(s): Commercialization of this technology will benefit the public health by increasing the number of therapeutics available for the public.

This Patent License Agreement, hereinafter referred to as the “**Agreement**”, consists of this Cover Page, an attached **Agreement**, a Signature Page, Appendix A (List of Patent(s) or Patent Application(s)), Appendix B (Fields of Use and Territory), Appendix C (Royalties), Appendix D ((Benchmarks and Performance), Appendix E (Commercial Development Plan), Appendix F (Example Royalty Report), and Appendix G (Royalty Payment Options). The Parties to this **Agreement** are:

- 1) The National Institutes of Health (“**NIH**”) or the Food and Drug Administration (“**FDA**”), hereinafter singly or collectively referred to as “**PHS**”, agencies of the United States Public Health Service within the Department of Health and Human Services (“**HHS**”); and
- 2) The person, corporation, or institution identified above and on the Signature Page, having offices at the address indicated on the Signature Page, hereinafter referred to as “**Licensee**.”

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**PHS PATENT LICENSE AGREEMENT-NONEXCLUSIVE**

**PHS** and **Licensee** agree as follows:

1. **BACKGROUND**

- 1.1 In the course of conducting biomedical and behavioral research, **PHS** investigators made inventions that may have commercial applicability.
- 1.2 By assignment of rights from **PHS** employees and other inventors, **HHS**, on behalf of the **Government**, owns intellectual property rights claimed in any United States or foreign patent applications or patents corresponding to the assigned inventions. **HHS** also owns any tangible embodiments of these inventions actually reduced to practice by **PHS**.
- 1.3 The Secretary of **HHS** has delegated to **PHS** the authority to enter into this **Agreement** for the licensing of rights to these inventions under 35 U.S.C. §§200-212, the Federal Technology Transfer Act of 1986, 15 U.S.C. §3710(a), and the regulations governing the licensing of Government-owned inventions, 37 CFR Part 404.

- 1.4 **PHS** desires to transfer these inventions to the private sector through commercialization licenses to facilitate the commercial development of products and processes for public use and benefit.
- 1.5 **Licensee** desires to acquire commercialization rights to certain of these inventions in order to develop processes, methods, or marketable products for public use and benefit.

## 2. DEFINITIONS

- 2.1 “**Benchmarks**” mean the performance milestones that are set forth in Appendix D.
- 2.2 “**Commercial Development Plan**” means the written commercialization plan attached as Appendix E.
- 2.3 “**First Commercial Sale**” means the initial transfer by or on behalf of **Licensee** or its sublicensees of **Licensed Products** or **New Products** by or on behalf of **Licensee** or its sublicensees in exchange for cash or some equivalent to which value can be assigned for the purpose of determining **Net Sales**.
- 2.4 “**Government**” means the **Government** of the United States of America.
- 2.5 “**Licensed Fields of Use**” means the fields of use identified in Appendix B.
- 2.6 “**Licensed Patent Rights**” shall mean:

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- (a) Patent applications (including provisional patent applications and PCT patent applications) or patents listed in Appendix A, all divisions and continuations of these applications, all patents issuing from these applications, divisions, and continuations, and any reissues, reexaminations, and extensions of all these patents;
- (b) to the extent that the following contain one or more claims directed to the invention or inventions disclosed in 2.6(a):
- (i) continuations-in-part of 2.6(a);
  - (ii) all divisions and continuations of these continuations-in-part;
  - (iii) all patents issuing from these continuations-in-part, divisions, and continuations;
  - (iv) priority patent application(s) of 2.6(a); and
  - (v) any reissues, reexaminations, and extensions of all these patents;
- (c) to the extent that the following contain one or more claims directed to the invention or inventions disclosed in 2.6(a): all counterpart foreign and U.S. patent applications and patents to 2.6(a) and 2.6(b), including those listed in Appendix A; and
- (d) **Licensed Patent Rights** shall *not* include 2.6(b) or 2.6(c) to the extent that they contain one or more claims directed to new matter which is not the subject matter disclosed in 2.6(a).
- 2.7 “**Licensed Processes**” means processes, which in the course of being practiced, would be within the scope of one or more claims of the **Licensed Patent Rights** that have not been held unpatentable, invalid or unenforceable by an unappealed or unappealable judgment of a court of competent jurisdiction.
- 2.8 “**Licensed Products**” means (a) **Supplied Materials** and (b) tangible materials, which in the course of manufacture, use, sale, or importation, would be within the scope of one or more claims of the **Licensed Patent Rights** that have not been held unpatentable, invalid or unenforceable by an unappealed or unappealable judgment of a court of competent jurisdiction.
- 2.9 “**Licensed Territory**” means the geographical area identified in Appendix B.
- 2.10 “**Net Sales**” means the total gross receipts for sales of **Licensed Products** or **New Products** by or on behalf of **Licensee** or its sublicensees, and from leasing, renting, or otherwise making **Licensed Products** or **New Products** available to others without sale or other dispositions, whether invoiced or not, less returns and allowances, packing costs, insurance costs, freight out, taxes or excise duties

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imposed on the transaction (if separately invoiced), and wholesaler and cash discounts in amounts customary in the trade to the extent actually granted. No deductions shall be made for commissions paid to individuals, whether they are with independent sales agencies or regularly employed by **Licensee** or its sublicensees, and on its payroll, or for the cost of collections.

- 2.11 “**New Product**” means a product made using a **Licensed Process** but excluding **Licensed Products**.
- 2.12 “**Practical Application**” means to manufacture in the case of a composition or product, to practice in the case of a process or method, or to operate in the case of a machine or system; and in each case, under these conditions as to establish that the invention is being utilized and that its benefits are to the extent permitted by law or **Government** regulations available to the public on reasonable terms.
- 2.13 **Supplied Materials**” means [\*\*]. Further, these **Supplied Materials** were supplied by **PHS** to **Licensee** under a Non-Exclusive Patent License Agreement for Internal Commercial Use (L-043-2003/0) which was effective on May 14, 2003.

3. GRANT OF RIGHTS

- 3.1 **PHS** hereby grants and **Licensee** accepts, subject to the terms and conditions of this **Agreement**, a nonexclusive license under the **Licensed Patent Rights** in the **Licensed Territory** to make and have made, to use and have used, to sell and have sold, to offer to sell, and to import any **Licensed Products** or **New Products** in the **Licensed Fields of Use**, to practice and have practiced any **Licensed Processes** in the **Licensed Fields of Use**, to make, have made, to use and have used but not to sell any **Supplied Materials**. As used in this **Agreement**, “have made” and “have used” means that **Licensee** shall have the limited right to use a third party contract manufacturer to make and use only (but not to sell) **Supplied Materials**, **Licensed Products** or **New Products**. **Licensee** acknowledges and agrees that any such third party contract manufacturer shall be bound to the terms and obligations of this **Agreement**.
- 3.2 This **Agreement** confers no license or rights by implication, estoppel, or otherwise under any patent applications or patents of **PHS** other than the **Licensed Patent Rights** regardless of whether these patents are dominant or subordinate to the **Licensed Patent Rights**.

4. SUBLICENSING

- 4.1 Upon written approval, which shall include prior review of any sublicense agreement by **PHS** and which shall not be unreasonably withheld, **Licensee** may enter into sublicensing agreements under the **Licensed Patent Rights**, except that

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**Licensee** shall not have the right to solely sublicense **Licensed Patent Rights**. For the avoidance of doubt, **Licensee** shall only sublicense the **Licensed Patent Rights** in conjunction with other intellectual property owned by the **Licensee** or in-licensed by the **Licensee**.

- 4.2 **Licensee** agrees that any sublicenses granted by it shall provide that the obligations to **PHS** of Paragraphs 5.1, 5.2, 8.1, 10.1, 10.2, 12.5 and 13.6-13.8 of this **Agreement** shall be binding upon the sublicensee as if it were a party to this **Agreement**. **Licensee** further agrees to attach copies of these Paragraphs to all sublicense agreements.
- 4.3 Any sublicenses granted by **Licensee** shall provide for the termination of the sublicense, or the conversion to a license directly between the sublicensee and **PHS**, at the option of the sublicensee, upon termination of this **Agreement** under Article 13. This conversion is subject to **PHS** approval and contingent upon acceptance by the sublicensee of the remaining provisions of this **Agreement**.
- 4.4 **Licensee** agrees to forward **PHS** a complete copy of each fully executed sublicense agreement postmarked within thirty (30) days of the execution of the agreement. To the extent permitted by law, **PHS** agrees to maintain each sublicense agreement in confidence.

5. STATUTORY AND PHS REQUIREMENTS AND RESERVED GOVERNMENT RIGHTS

- 5.1 Prior to the **First Commercial Sale**, **Licensee** agrees to provide **PHS** with reasonable quantities of **Licensed Products** or **New Products** made through the **Licensed Processes** or **Supplied Materials** solely for **PHS** research use, if requested in writing.
- 5.2 **Licensee** agrees that products used or sold in the United States embodying **Licensed Products** or **New Products** or produced through use of **Licensed Processes** shall be manufactured substantially in the United States, unless a written waiver is obtained in advance from **PHS**.

6. ROYALTIES AND REIMBURSEMENT

- 6.1 **Licensee** agrees to pay **PHS** a noncreditable, nonrefundable license issue royalty as set forth in Appendix C.
- 6.2 **Licensee** agrees to pay **PHS** a nonrefundable minimum annual royalty as set forth in Appendix C.
- 6.3 **Licensee** agrees to pay **PHS** earned royalties as set forth in Appendix C.
- 6.4 **Licensee** agrees to pay **PHS** sublicensing royalties as set forth in Appendix C.

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- 6.5 A patent or patent application licensed under this **Agreement** shall cease to fall within the **Licensed Patent Rights** for the purpose of computing earned royalty payments in any given country on the earliest of the dates that:
- (a) the application has been abandoned and not continued;
  - (b) the patent expires or irrevocably lapses; or
  - (c) the claim has been held to be invalid or unenforceable by an unappealed or unappealable decision of a court of competent jurisdiction or administrative agency.
- 6.6 No multiple royalties shall be payable because any **Licensed Products** or **New Products** or **Licensed Processes** are covered by more than one of the **Licensed Patent Rights**.
- 6.7 On sales of **Licensed Products** or **New Products** by **Licensee** to sublicensees or on sales made in other than an arms-length transaction, the value of the **Net Sales** attributed under this Article 6 to this transaction shall be that which would have been received in an arms-length transaction, based on sales of like quantity and quality products on or about the time of this transaction.

7. PATENT FILING, PROSECUTION, AND MAINTENANCE

7.1 **PHS** agrees to take responsibility for the preparation, filing, prosecution, and maintenance of any and all patent applications or patents included in the **Licensed Patent Rights**.

## 8. RECORD KEEPING

8.1 **Licensee** agrees to keep accurate and correct records of **Licensed Products** or **New Products** made, used, sold, or imported and **Licensed Processes** practiced under this **Agreement** appropriate to determine the amount of royalties due **PHS**. These records shall be retained for at least **[\*\*]** years following a given reporting period and shall be available during normal business hours for inspection, at the expense of **PHS**, by an accountant or other designated auditor selected by **PHS** for the sole purpose of verifying reports and royalty payments hereunder. The accountant or auditor shall only disclose to **PHS** information relating to the accuracy of reports and royalty payments made under this **Agreement**. If an inspection shows an underreporting or underpayment in excess of **[\*\*]** percent (**[\*\*]**%) for any **[\*\*]** month period, then **Licensee** shall reimburse **PHS** for the cost of the inspection at the time **Licensee** pays the unreported royalties, including any additional royalties as required by Paragraph 9.8. All royalty payments required under this Paragraph shall be due within **[\*\*]** days of the date **PHS** provides **Licensee** notice of the payment due.

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8.2 **Licensee** agrees to have an audit of sales and royalties conducted by an independent auditor at least every **[\*\*]** years if annual sales of the **Licensed Products** or **Licensed Processes** or **New Products** are over **[\*\*]** dollars. The audit shall address, at a minimum, the amount of gross sales by or on behalf of **Licensee** during the audit period, terms of the license as to percentage or fixed royalty to be remitted to the **Government**, the amount of royalties owed to the **Government** under this **Agreement**, and whether the royalties owed have been paid to the **Government** and is reflected in the records of the **Licensee**. The audit shall also indicate the **PHS** license number, product, and the time period being audited. A report certified by the auditor shall be submitted promptly by the auditor directly to **PHS** and **Licensee** on completion.

## 9. REPORTS ON PROGRESS, BENCHMARKS, SALES, AND PAYMENTS

9.1 Prior to signing this **Agreement**, **Licensee** has provided **PHS** with the **Commercial Development Plan** in Appendix E, under which **Licensee** intends to bring the subject matter of the **Licensed Patent Rights** or **New Products** to the point of **Practical Application**. This **Commercial Development Plan** is hereby incorporated by reference into this **Agreement**. Based on this plan, performance **Benchmarks** are determined as specified in Appendix D.

9.2 **Licensee** shall provide written annual reports on its product development progress or efforts to commercialize under the **Commercial Development Plan** for the **Licensed Fields of Use** within **[\*\*]** days after December 31 of each calendar year. These progress reports shall include, but not be limited to: progress on research and development, status of applications for regulatory approvals, manufacturing, marketing, importing, and sales during the preceding calendar year, as well as, plans for the present calendar year. **PHS** also encourages these reports to include information on any of **Licensee's** public service activities that relate to the **Licensed Patent Rights** or **New Products**. If reported progress differs from that projected in the **Commercial Development Plan** and **Benchmarks**, **Licensee** shall explain the reasons for such differences. In any annual report, **Licensee** may propose amendments to the **Commercial Development Plan**, acceptance of which by **PHS** may not be denied or delayed unreasonably. **Licensee** agrees to provide any additional information reasonably required by **PHS** to evaluate **Licensee's** performance under this **Agreement**. **Licensee** may amend the **Benchmarks** at any time upon written approval by **PHS**. **PHS** shall not unreasonably withhold approval of any request of **Licensee** to extend the time periods of this schedule if the request is supported by a reasonable showing by **Licensee** of diligence in its performance under the **Commercial Development Plan** and toward bringing the **Licensed Products** or **New Products** to the point of **Practical Application**.

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9.3 **Licensee** shall report to **PHS** the dates for achieving **Benchmarks** specified in Appendix D and the **First Commercial Sale** in each country in the **Licensed Territory** within **[\*\*]** days of such occurrences.

9.4 **Licensee** shall submit to **PHS**, within **[\*\*]** days after each calendar half-year ending June 30 and December 31, a royalty report, as described in the example in Appendix F, setting forth for the preceding half-year period the amount of the **Licensed Products** or **New Products** sold or **Licensed Processes** practiced by or on behalf of **Licensee** in each country within the **Licensed Territory**, the Net Sales, and the amount of royalty accordingly due. With each royalty report, **Licensee** shall submit payment of earned royalties due. If no earned royalties are due to **PHS** for any reporting period, the written report shall so state. The royalty report shall be certified as correct by an authorized officer of **Licensee** and shall include a detailed listing of all deductions made under Paragraph 2.10 to determine **Net Sales** made under Article 6 to determine royalties due.

9.5 **Licensee** agrees to forward semi-annually to **PHS** a copy of reports received by **Licensee** from its sublicensees during the preceding half-year period as shall be pertinent to a royalty accounting to **PHS** by **Licensee** for activities under the sublicense.

9.6 Royalties due under Article 6 shall be paid in U.S. dollars and payment options are listed in Appendix G. For conversion of foreign currency to U.S. dollars, the conversion rate shall be the New York foreign exchange rate quoted in *The Wall Street Journal* on the day that the payment is due, and any loss of exchange, value, taxes, or other expenses incurred in the transfer or conversion to U.S. dollars shall be paid entirely by **Licensee**. The royalty report required by Paragraph 9.4 shall be mailed to **PHS** at its address for **Agreement** Notices indicated on the Signature Page.

9.7 **Licensee** shall be solely responsible for determining if any tax on royalty income is owed outside the United States and shall pay this tax and be responsible for all filings with appropriate agencies of foreign governments.

9.8 Additional royalties may be assessed by **PHS** on any payment that is more than **[\*\*]** days overdue at the rate of **[\*\*]** percent (**[\*\*]**%) per month. This **[\*\*]** percent (**[\*\*]**%) per month rate may be applied retroactively from the original due date until the date of receipt by **PHS** of the overdue payment and additional royalties. The payment of any additional royalties shall not prevent **PHS** from exercising any other rights it may have as a consequence of the lateness of any payment.

confidential, and any proposed disclosure of these records by the **PHS** under the Freedom of Information Act (FOIA), 5 U.S.C. §552 shall be subject to the predisclosure notification requirements of 45 CFR §5.65(d).

10. PERFORMANCE

- 10.1 **Licensee** shall use its reasonable commercial efforts to bring the **Licensed Products** or **New Products** and **Licensed Processes** to **Practical Application**. “Reasonable commercial efforts” for the purposes of this provision shall include adherence to the **Commercial Development Plan** in Appendix E and performance of the **Benchmarks** in Appendix D. The efforts of a sublicense shall be considered the efforts of **Licensee**.
- 10.2 Upon the **First Commercial Sale**, until the expiration or termination of this **Agreement**, **Licensee** shall use its reasonable commercial efforts to make **Licensed Products** or **New Products** and **Licensed Processes** reasonably accessible to the United States public.
- 10.3 **Licensee** agrees, after its **First Commercial Sale**, to make reasonable quantities of **Licensed Products** or **New Products** or materials produced through the use of **Licensed Processes** available on a compassionate use basis to patients, either through the patient’s physician(s) or the medical center treating the patient.
- 10.4 **Licensee** agrees, after its **First Commercial Sale** and as part of its marketing and product promotion, to develop educational materials (e.g., brochures, website, etc.) directed to patients and physicians detailing the **Licensed Products** or **New Products** or medical aspects of the prophylactic and therapeutic uses of the **Licensed Products** or **New Products**.
- 10.5 **Licensee** agrees to supply, to the Mailing Address for **Agreement** Notices indicated on the Signature Page, the Office of Technology Transfer, **NIH** with inert samples of the **Licensed Products** or **New Products** or their packaging for educational and display purposes only.

11. INFRINGEMENT AND PATENT ENFORCEMENT

- 11.1 **PHS** and **Licensee** agree to notify each other promptly of each infringement or possible infringement of the **Licensed Patent Rights**, as well as, any facts which may affect the validity, scope, or enforceability of the **Licensed Patent Rights** of which either Party becomes aware.
- 11.2 In the event that a declaratory judgment action alleging invalidity of any of the **Licensed Patent Rights** shall be brought against **PHS**, **PHS** agrees to notify **Licensee** that an action alleging invalidity has been brought. **PHS** does not represent that it shall commence legal action to defend against a declaratory action alleging invalidity. **Licensee** shall take no action to compel the

**Government** either to initiate or to join in any declaratory judgment action. Should the **Government** be made a party to any suit by motion or any other action of **Licensee**, **Licensee** shall reimburse the **Government** for any costs, expenses, or fees, which the **Government** incurs as a result of the motion or other action. Upon **Licensee’s** payment of all costs incurred by the **Government** as a result of **Licensee’s** joinder motion or other action, these actions by **Licensee** shall not be considered a default in the performance of any material obligation under this **Agreement**.

12. NEGATION OF WARRANTIES AND INDEMNIFICATION

- 12.1 **PHS** offers no warranties other than those specified in Article 1.
- 12.2 **PHS** does not warrant the validity of the **Licensed Patent Rights** and makes no representations whatsoever with regard to the scope of the **Licensed Patent Rights**, or that the **Licensed Patent Rights** and **Supplied Materials** may be exploited without infringing other patents or other intellectual property rights of third parties.
- 12.3 **PHS** MAKES NO WARRANTIES, EXPRESSED OR IMPLIED, OF MERCHANTABILITY OR FITNESS FOR A PARTICULAR PURPOSE OF ANY SUBJECT MATTER DEFINED BY THE CLAIMS OF THE **LICENSED PATENT RIGHTS** OR TANGIBLE MATERIALS RELATED THERETO, INCLUDING BUT NOT LIMITED TO **SUPPLIED MATERIALS**.
- 12.4 **PHS** does not represent that it shall commence legal actions against third parties infringing the **Licensed Patent Rights** or **Supplied Materials**.
- 12.5 **Licensee** shall indemnify and hold **PHS**, its employees, students, fellows, agents, and consultants harmless from and against all liability, demands, damages, expenses, and losses, including but not limited to death, personal injury, illness, or property damage in connection with or arising out of:
- (a) the use by or on behalf of **Licensee**, its sublicensees, its directors, employees, or third parties of any **Licensed Patent Rights** or **Supplied Materials**; or
  - (b) the design, manufacture, distribution, or use of any **Licensed Products**, **Licensed Processes** or **New Products** by **Licensee**, or other products or processes developed in connection with or arising out of the **Licensed Patent Rights**. **Licensee** agrees to maintain a liability insurance program consistent with sound business practice.
- 12.6 **Licensee** agrees to maintain a liability insurance program consistent with sound business practice.

13. TERM, TERMINATION, AND MODIFICATION OF RIGHTS

- 13.1 This **Agreement** is effective when signed by all parties, unless the provisions of Paragraph 14.15 are not fulfilled, and shall extend to the expiration of the last to expire of the **Licensed Patent Rights** unless sooner terminated as provided in this Article 13.
- 13.2 In the event that **Licensee** is in default in the performance of any material obligations under this **Agreement**, including but not limited to the obligations listed in Paragraph 13.05, and if the default has not been remedied within **[\*\*]** days after the date of notice in writing of the default, **PHS** may terminate this **Agreement** by written notice and pursue outstanding royalties owed through procedures provided by the Federal Debt Collection Act.
- 13.3 In the event that **Licensee** becomes insolvent, files a petition in bankruptcy, has such a petition filed against it, determines to file a petition in bankruptcy, or receives notice of a third party's intention to file an involuntary petition in bankruptcy, **Licensee** shall immediately notify **PHS** in writing. Furthermore, **PHS** shall have the right to terminate this **Agreement** immediately upon **Licensee's** receipt of written notice.
- 13.4 **Licensee** shall have a unilateral right to terminate this **Agreement** in any country or territory by giving **PHS** sixty (60) days written notice to that effect.
- 13.5 **PHS** shall specifically have the right to terminate or modify, at its option, this **Agreement**, if **PHS** determines that the **Licensee**:
- (a) is not executing the **Commercial Development Plan** submitted with its request for a license and the **Licensee** cannot otherwise demonstrate to **PHS'** satisfaction that the **Licensee** has taken, or can be expected to take within a reasonable time, effective steps to achieve **Practical Application** of the **Licensed Products** or **New Products**;
  - (b) has not achieved the **Benchmarks** as may be modified under Paragraph 9.2;
  - (c) has willfully made a false statement of, or willfully omitted, a material fact in the license application or in any report required by this **Agreement**;
  - (d) has committed a material breach of a covenant or agreement contained in this **Agreement**;
  - (e) is not keeping **Licensed Products** or **New Products** reasonably available to the public after commercial use commences;
  - (f) cannot reasonably satisfy unmet health and safety needs; or

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- (g) cannot reasonably justify a failure to comply with the domestic production requirement of Paragraph 5.2, unless waived.
- 13.6 In making the determination referenced in Paragraph 13.5, **PHS** shall take into account the normal course of such commercial development programs conducted with sound and reasonable business practices and judgment and the annual reports submitted by **Licensee** under Paragraph 9.2. Prior to invoking termination or modification of this **Agreement** under Paragraph 13.5, **PHS** shall give written notice to **Licensee** providing **Licensee** specific notice of, and a **[\*\*]** day opportunity to respond to, **PHS'** concerns as to the items referenced in 13.5(a)-13.5(g). If **Licensee** fails to alleviate **PHS'** concerns as to the items referenced in 13.5(a)-13.5(g) or fails to initiate corrective action to **PHS'** satisfaction, **PHS** may terminate this **Agreement**.
- 13.7 **PHS** reserves the right according to 35 U.S.C. §209(d)(3) to terminate or modify this **Agreement** if it is determined that the action is necessary to meet the requirements for public use specified by federal regulations issued after the date of the license and these requirements are not reasonably satisfied by **Licensee**.
- 13.8 Within **[\*\*]** days of receipt of written notice of **PHS'** unilateral decision to modify or terminate this **Agreement**, **Licensee** may, consistent with the provisions of 37 CFR §404.11, appeal the decision by written submission to the designated **PHS** official. The decision of the designated **PHS** official shall be the final agency decision. **Licensee** may thereafter exercise any and all administrative or judicial remedies that may be available.
- 13.9 Within **[\*\*]** days of expiration or termination of this **Agreement** under this Article 13, a final report shall be submitted by **Licensee**. Any royalty payments, including those incurred but not yet paid (such as the full minimum annual royalty), and those related to patent expense, due to **PHS** shall become immediately due and payable upon termination or expiration. If terminated under this Article 13, sublicensees may elect to convert their sublicenses to direct licenses with **PHS** pursuant to Paragraph 4.3. Unless otherwise specifically provided for under this **Agreement**, upon termination or expiration of this **Agreement**, **Licensee** shall return all **Licensed Products** and **New Products** or other materials included within the **Licensed Patent Rights** and under its control to **PHS** or provide **PHS** with written certification of the destruction thereof.

14. GENERAL PROVISIONS

- 14.1 Neither party may waive or release any of its rights or interests in this **Agreement** except in writing. The failure of the **Government** to assert a right hereunder or to insist upon compliance with any term or condition of this **Agreement** shall not

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constitute a waiver of that right by the **Government** or excuse a similar subsequent failure to perform any of these terms or conditions by **Licensee**.

- 14.2 This **Agreement** constitutes the entire **Agreement** between the Parties relating to the subject matter of the **Licensed Patent Rights, Licensed Products, New Products, Supplied Materials** and **Licensed Processes**, and all prior negotiations, representations, agreements, and understandings are merged into, extinguished by, and completely expressed by this **Agreement**.
- 14.3 The provisions of this **Agreement** are severable, and in the event that any provision of this **Agreement** shall be determined to be invalid or unenforceable under any controlling body of law, this determination shall not in any way affect the validity or enforceability of the remaining provisions of this **Agreement**.
- 14.4 If either party desires a modification to this **Agreement**, the parties shall, upon reasonable notice of the proposed modification by the party desiring the change, confer in good faith to determine the desirability of the modification. No modification shall be effective until a written amendment is signed by the signatories to this **Agreement** or their designees.
- 14.5 The construction, validity, performance, and effect of this **Agreement** shall be governed by Federal law as applied by the Federal courts in the District of Columbia.
- 14.6 All **Agreement** notices required or permitted by this **Agreement** shall be given by prepaid, first class, registered or certified mail or by an express/overnight delivery service provided by a commercial carrier, properly addressed to the other party at the address designated on the Signature Page, or to any other address as may be designated in writing by such other party. **Agreement** notices shall be considered timely if such notices are received on or before the established deadline date or sent on or before the deadline date as verifiable by U.S. Postal Service postmark or dated receipt from a commercial carrier. Parties should request a legibly dated U.S. Postal Service postmark or obtain a dated receipt from a commercial carrier or the U.S. Postal Service. Private metered postmarks shall not be acceptable as proof of timely mailing.
- 14.7 This **Agreement** shall not be assigned by **Licensee** except:
- (a) with the prior written consent of **PHS**, this consent shall not be withheld unreasonably; or
  - (b) as part of a sale or transfer of substantially the entire business of **Licensee** relating to operations which concern this **Agreement**; and
  - (c) **Licensee** shall notify **PHS** within **[\*\*]** days of any assignment of this **Agreement** by **Licensee**, and **Licensee** shall pay **PHS**, as an additional

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royalty, **[\*\*]** percent of the fair market value of any consideration received for any assignment of this **Agreement** within **[\*\*]** days of the assignment.

- 14.8 **Licensee** agrees in its use of any **Supplied Materials** to comply with all applicable statutes, regulations, and guidelines, including **PHS** and **HHS** regulations and guidelines. **Licensee** agrees not to use the **Supplied Materials** for research involving human subjects or clinical trials in the United States without complying with 21 CFR Part 50 and 45 CFR Part 46. **Licensee** agrees not to use the **Supplied Materials** for research involving human subjects or clinical trials outside of the United States without notifying **PHS**, in writing, of the research or trials and complying with the applicable regulations of the appropriate national control authorities. Written notification to **PHS** of research involving human subjects or clinical trials outside of the United States shall be given no later than **[\*\*]** days prior to commencement of the research or trials.
- 14.9 **Licensee** acknowledges that it is subject to and agrees to abide by the United States laws and regulations (including the Export Administration Act of 1979 and Arms Export Control Act) controlling the export of technical data, computer software, laboratory prototypes, biological materials, **Supplied Materials** and other commodities. The transfer of these items may require a license from the appropriate agency of the **Government** or written assurances by **Licensee** that it shall not export these items to certain foreign countries without prior approval of the agency. **PHS** neither represents that a license is or is not required or that, if required, it shall be issued.
- 14.10 **Licensee** agrees to mark the **Licensed Products** or **New Products** or their packaging sold in the United States with all applicable U.S. patent numbers and similarly to indicate "Patent Pending" status. All **Licensed Products** or **New Products** manufactured in, shipped to, or sold in other countries shall be marked in a manner to preserve **PHS** patent rights in those countries.
- 14.11 By entering into this **Agreement**, **PHS** does not directly or indirectly endorse any product or service provided, or to be provided, by **Licensee** whether directly or indirectly related to this **Agreement**. **Licensee** shall not state or imply that this **Agreement** is an endorsement by the **Government**, **PHS**, any other **Government** organizational unit, or any **Government** employee. Additionally, **Licensee** shall not use the names of **NIH**, **PHS**, **FDA** or **HHS** or the **Government** or their employees in any advertising, promotional, or sales literature without the prior written approval of **PHS**.
- 14.12 The Parties agree to attempt to settle amicably any controversy or claim arising under this **Agreement** or a breach of this **Agreement**, except for appeals of modifications or termination decisions provided for in Article 13. **Licensee**

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agrees first to appeal any unsettled claims or controversies to the designated **PHS** official, or designee, whose decision shall be considered the final agency decision. Thereafter, **Licensee** may exercise any administrative or judicial remedies that may be available.

- 14.13 Nothing relating to the grant of a license, nor the grant itself, shall be construed to confer upon any person any immunity from or defenses under the antitrust laws or from a charge of patent misuse, and the acquisition and use of rights pursuant to 37 CFR Part 404 shall not be immunized from the operation of state or Federal law by reason of the source of the grant.
- 14.14 Paragraphs 8.1, 9.7-9.9, 12.1-12.5, 13.8, 13.9, 14.12 and 14.14 of this **Agreement** shall survive termination of this **Agreement**.



14.15 The terms and conditions of this **Agreement** shall, at **PHS**' sole option, be considered by **PHS** to be withdrawn from **Licensee's** consideration and the terms and conditions of this **Agreement**, and the **Agreement** itself to be null and void, unless this **Agreement** is executed by the **Licensee** and a fully executed original is received by **PHS** within [\*\*] days from the date of **PHS** signature found at the Signature Page.

**SIGNATURES BEGIN ON NEXT PAGE**

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**PHS PATENT LICENSE AGREEMENT — NONEXCLUSIVE**

**SIGNATURE PAGE**

For **PHS**:

/s/ Steven M. Ferguson

4/25/07

Steven M. Ferguson

Date

Director, Division of Technology Development and Transfer  
Office of Technology Transfer  
National Institutes of Health

Mailing Address for **Agreement** notices:

Chief, Monitoring & Enforcement Branch  
Office of Technology Transfer  
National Institutes of Health  
6011 Executive Boulevard, Suite 325  
Rockville, Maryland 20852-3804 U.S.A.

For **Licensee** (Upon, information and belief, the undersigned expressly certifies or affirms that the contents of any statements of **Licensee** made or referred to in this document are truthful and accurate.):

by:

/s/ Ronald H.W. Lorijn

5/2/07

Signature of Authorized Official

Date

Ronald H.W. Lorijn

Printed Name

C.E.O.

Title

I. Official and Mailing Address for **Agreement** notices:

Sander van Deventer, M.D.  
Chief Scientific Officer  
Amsterdam Molecular Therapeutics  
Meibergdreef 61  
P.O. Box 22506  
1100DA Amsterdam, Netherlands

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II. Official and Mailing Address for Financial notices (**Licensee's** contact person for royalty payments)

Sander van Deventer, M.D.

Name

Chief Scientific Officer

Title

Mailing Address:

Amsterdam Molecular Therapeutics  
Meibergdreef 61  
P.O. Box 22506  
1100DA Amsterdam, Netherlands

Email Address: s.vandeventer@amtbv.com

Phone: +31-20-5669272

Fax: +31-20-5669272

**APPENDIX A - PATENT(S) OR PATENT APPLICATION(S)**

**Patent(s) or Patent Application(s):**

I. U.S. Patent Application(s) or Patent(s):

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II. PCT or Foreign Patent Application(s) or Patent(s):

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**APPENDIX B - LICENSED FIELDS OF USE AND TERRITORY**

I. **Licensed Fields of Use:**

Use of the **Licensed Patent Rights** for the commercial development of AAV related products within the scope of the **Agreement**.

II. **Licensed Territory:**

United States, Australia, Canada and Europe.

**APPENDIX C - ROYALTIES**

**Royalties:**

I. **Licensee** agrees to pay to **PHS** a noneritable, nonrefundable license issue royalty in the amount of twelve thousand U.S. dollars (\$12,000) within [\*\*] days from the effective date of this **Agreement**.

II. **Licensee** agrees to pay to **PHS** a nonrefundable minimum annual royalty in the amount of [\*\*] U.S. dollars (\$[\*\*]) as follows:

- (a) The first minimum annual royalty is due within [\*\*] days of the effective date of this **Agreement** and may be prorated according to the fraction of the calendar year remaining between the effective date of this **Agreement** and the next subsequent January 1; and
- (b) Subsequent minimum annual royalty payments are due and payable on January 1 of each calendar year and may be credited against any earned royalties due for sales made in that year.

III. **Licensee** agrees to pay **PHS** earned royalties of [\*\*] percent ([\*\*]%) on **Net Sales** by or on behalf of **Licensee** for **Licensed Products**.

IV. **Licensee** agrees to pay **PHS** earned royalties of [\*\*] percent ([\*\*]%) on **Net Sales** by or on behalf of **Licensee** for **New Products**.

V. **Licensee** agrees to pay **PHS** Benchmark royalties within [\*\*] days of achieving each **Benchmark**:

- (a) [\*\*] U.S. dollars (\$[\*\*]) — Initiation of each Phase I clinical trial or foreign equivalent.
- (b) [\*\*] U.S. dollars (\$[\*\*]) — Initiation of each Phase II clinical trial or foreign equivalent.
- (c) [\*\*] U.S. dollars (\$[\*\*]) — Initiation of each Phase III clinical trial or foreign equivalent.
- (d) Initiation of first Marketing Approval or foreign equivalent in the following jurisdictions/countries:
  - (i) [\*\*] U.S. dollars (\$[\*\*])
  - (ii) [\*\*] U.S. dollars (\$[\*\*]).
  - (iii) [\*\*] U.S. dollars (\$[\*\*]).
  - (iv) [\*\*] U.S. dollars (\$[\*\*]).

IV. **Licensee** agrees to pay **PHS** additional sublicensing royalties as follows:

[\*\*] percent ([\*\*]%) of the fair market value of any consideration received for granting each sublicense.

## APPENDIX D - BENCHMARKS AND PERFORMANCE

**Licensee** agrees to the following **Benchmarks** for its performance under this **Agreement** and, within [\*\*] days of achieving a **Benchmark**, shall notify **PHS** that the **Benchmark** has been achieved.

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## APPENDIX E - COMMERCIAL DEVELOPMENT PLAN

### Introduction

In 2004 the European Regulatory Authority (“**EMEA**”), assigned the ‘orphan drug’ status to **Licensee’s** lead product AMT-010. AMT-010 (Adeno-Associated Viral Vector Expressing Human Lipoprotein LipaseS447X), a gene therapy product to treat Lipoprotein Lipase Deficiency Type I and V deficiency, has entered its phase I/II clinical stage. This program is focused on treating the rare, but serious and disabling inherited disease called LPL type 1 deficiency for which no adequate treatment exists today.

Due to a substantial change in the manufacturing process (from DKFZ to Baculo), **Licensee** does not intend to market AMT-010 but instead the newly produced product AMT-011.

For that reason, **Licensee** has started preclinical development in 2006, and will initiate a phase II clinical study with AMT-011 in Canada in Q3 2007, as well as a phase II clinical study for type V hyperlipoproteinemia in Q4 2007. **Licensee** expects to file the registration dossier for type I hyperlipoproteinemia with the **EMEA** by Q1 2008.

Early 2007, **Licensee** will begin discussions with the FDA to prepare the filing of the AMT-011 dossier for registration in the U.S.A. Depending on the feedback from the U.S. authorities, **Licensee** plans to file its registration dossier with the FDA in 2008.

### Technology

**Licensee** is building gene therapies using adeno-associated viral (AAV)-based vectors. These vectors do not integrate into the host genome and result in long-lasting expression of therapeutic genes. AAV vectors can be specifically targeted to various organs (i.e. muscle, brain, liver, retina) and even to specific cells within a target organ. Improvements in local expression are also achieved by using potent organ specific promoters. **Licensee** has extensively optimized expression of therapeutic genes in various organs by selection of AAV serotype-promoter combinations and by selection of high expressing transgenes. In relevant preclinical models, this has resulted in lifelong (2 years for mouse and rat) expression of transgenes at therapeutic levels (often 100% of the normal expression) and a complete cure of the disease. Because of this extensive knowledge base, and the availability of all relevant currently available AAV serotypes, **Licensee** believes it is in the position to rapidly develop genetic therapies for a wide range of diseases caused by single gene defects.

### The Disease: Lipoprotein Lipase Deficiency

Genetic lipoprotein lipase (LPL) deficiency results in profound hypertriglyceridemia, which is associated with intense chronic abdominal pain, hepatosplenomegaly, eruptive xanthomas, lipemia retinalis, dyspnea, mono- or polyparesthesias, and memory loss. Prolonged elevations in plasma triglycerides (TG) also induce recurrent episodes of often lethal pancreatitis, chronic pancreatic insufficiency, and diabetes mellitus. Currently, no effective treatment for this disease

exists. Patients must follow a strict low-fat diet. However, TG levels often remain above the critical threshold. Genetic LPL deficiency type I is a rare, autosomal recessive trait. Prevalence varies between 1 in 1,000,000 in the general population to 1 in 5,000 in French Quebec (a ‘founder effect’).

LPL gene therapy will also be investigated to treat type V hyperlipoproteinemia (prevalence of 1.8 in 10,000). LPL gene therapy may improve the quality of life and reduce the risk of morbidity and mortality for a significant number of patients that suffer from this particular lipid disorder.

Currently, no treatment for hyperlipoproteinemia is available, and patients suffer from repeated bouts of pancreatitis. LPL enzyme replacement therapy is not feasible in view of the very short half-life of the enzyme. The only advice a physician can give these patients is to keep a strict fat-free diet, which is extremely difficult to maintain. Further, even with such a diet, the serum triglyceride levels remain far above the critical level of 10 µmol/L.

### Business Strategy

**Licensee’s** core strategy will be to position the new medicine as an orphan drug. This strategy has several important benefits. The Regulatory Authorities in many countries including the EU (**EMEA**) and the U.S.A. (FDA) have recognized the significance of the development of orphan drugs. Thanks to the regulatory laws and regulations the ‘time-to-market’, IP and marketing rights protection are very favorable for such products.

Marketing exclusivity for orphan drugs after marketing authorization are:

- EU: 10 years
- USA: 7 years

This means that during that period no other sponsor can obtain marketing authorization for a similar product in the designated indication. **Licensee** received the orphan drug status for the LPL gene therapy from the EMEA last year. Due to these circumstances and the fact that the major pharmaceutical companies have little or no interest in developing products for these niche markets, the opportunities for companies such as **Licensee** are significant.

In the specific case for the LPL product, there is yet another element that will ensure a quick uptake and fast penetration in the target market. Specifically, there is no treatment today nor in the foreseeable future for patients suffering from LPL Type 1 and V deficiency. In other words, no major competing treatments are available to these patients.

In conclusion, no cure or symptomatic treatment, which would alleviate the disease’s symptoms and its complications exist today. The LPL project is unique because it exploits for the first time the possibility to treat patients suffering from LPL deficiency type I and V.

Marketing and Sales

Market Overview AMT-011

Lipoprotein lipase deficiency Type I	Hypertriglyceridemia Type V
· Orphan indication	· Larger indication
· 3,000-4,000 patients world-wide; very well organized and active patients groups	· 30 % of the hypertriglyceridemia patients have underlying LPL deficiency
· Estimated global sales \$ 200 M	· Estimated global sales >\$500 M
· No competition	· Competition: small molecule approaches

**Licensee’s** business strategy focuses on market entry for its orphan indications through its own dedicated marketing and sales force in Europe and North America. In the Western world, many patients suffering from serious orphan diseases have formed patient groups. Also their treating physicians, in many instances, are well connected. After marketing approval has been obtained, this situation allows for a very concentrated educational effort to inform patients and physicians about the important benefits of gene therapy. In order to ethically justify and successfully penetrate such markets, **Licensee** will ensure to build-up a highly educated medical service team to assist physicians in the selection of those patients who will benefit from the treatment. Such a team can be relatively small, which allows management to monitor and guide it closely.

**APPENDIX F - EXAMPLE ROYALTY REPORT**

**Required royalty report information includes:**

- OTT license reference number (L-XXX-200X/0)
- Reporting period
- Catalog number and units sold of each Licensed Product (domestic and foreign)
- Gross Sales per catalog number per country
- Total Gross Sales
- Itemized deductions from Gross Sales
- Total Net Sales
- Earned Royalty Rate and associated calculations
- Gross Earned Royalty
- Adjustments for Minimum Annual Royalty (MAR) and other creditable payments made
- Net Earned Royalty due

**Example**

Catalog Number	Product Name	Country	Units Sold	Gross Sales (US\$)
1	A	[**]	[**]	[**]
1	A	[**]	[**]	[**]
1	A	[**]	[**]	[**]
2	B	[**]	[**]	[**]
3	C	[**]	[**]	[**]
4	D	[**]	[**]	[**]
Total Gross Sales				[**]
Less Deductions:				
Freight				[**]
Returns				[**]
Total Net Sales				[**]
Royalty Rate				[**]
Royalty Due				[**]
Less Creditable Payments				[**]
Net Royalty Due				[**]

**NIH/PHS License Agreements**

**\*In order to process payment via Electronic Funds Transfer sender MUST supply the following information:**

**Procedure for Transfer of Electronic Funds to NIH for Royalty Payments**

Bank Name: Federal Reserve Bank

ABA# 021030004

TREAS NYC

BNF=/AC-75080031

OBI=Licensee Name and OTT Reference Number

Dollar Amount Wired=\$\$

NOTE: Only U.S. banks can wire directly to the Federal Reserve Bank. Foreign banks cannot wire directly to the Federal Reserve Bank, but must go through an intermediary U.S. bank. Foreign banks may send the wire transfer to the U.S. bank of their choice, who, in turn forwards the wire transfer to the Federal Reserve Bank.

**Mailing Address for Royalty Payments:**

National Institutes of Health

P.O. Box 360120

Pittsburgh, PA 15251-6120 USA

**Overnight Mail for Royalty Payments only**

National Institutes of Health

360120

Mellon Client Service Center

Room 670

500 Ross Street

Pittsburgh, PA 15262-0001

(412) 234-4381 (Customer Service)

**Please make checks payable to: NIH/Patent Licensing**

**The OTT Reference Number MUST appear on checks, reports and correspondence**

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**PUBLIC HEALTH SERVICE**

**FIRST AMENDMENT TO L-107-2007/0**

This is the first amendment (“**First Amendment**”) of the agreement by and between the National Institutes of Health (“**NIH**”) or the Food and Drug Administration (“**FDA**”), hereinafter singly or collectively referred to as agencies of the United States Public Health Service (“**PHS**”) within the Department of Health and Human Services (“**HHS**”), and Amsterdam Molecular Therapeutics having an effective date of May 2, 2007 and having **NIH** Reference Number L-107-2007/0 (“**Agreement**”). This **First Amendment**, having **NIH** Reference Number L-107-2007/1, is made between the **PHS** through the Office of Technology Transfer, **NIH**, having an address at 6011 Executive Boulevard, Suite 325, Rockville, Maryland 20852-3804, U.S.A., and Amsterdam Molecular Therapeutics, having an office at Meibergdreef 61, 1005 BA Amsterdam, The Netherlands (“**Licensee**”). This **First Amendment** includes, in addition to the amendments made below, 1) a Signature Page, 2) **Appendix D — Benchmarks and Performance**, 3) **Appendix E — Commercial Development Plan**, 4) Exhibit A — Request to Collaborate with Institute Pasteur, 5) Exhibit B- PHS Consent for Institute Pasteur Exemption, and 6) Attachment 1 (Royalty Payment Information).

WHEREAS, **Licensee** has requested an amendment to revise the **Benchmarks** for its lead **Licensed Product** AMT-011, also known as Glybera™;

WHEREAS, **Licensee** did not conduct a Phase III clinical trial for Glybera™ and has filed for Marketing Approval for Glybera™ with the European Medicines Agency on January 11, 2010;

WHEREAS, **Licensee** has requested **Benchmark** royalty exemption for collaborations with not-for-profit organizations and academic institutions for pre-clinical and clinical development to treat ultra-orphan indications;

WHEREAS, **PHS** requested that **Licensee** amend the **Appendix E - Commercial Development Plan** to state its development plans for **Licensed Products** other than Glybera™;

WHEREAS, **PHS** requested that **Licensee** add **Benchmarks** for **Licensed Products** other than Glybera™ to **Appendix D - Benchmarks and Performance**;

WHEREAS, **PHS** and **Licensee** desire that the **Agreement** be amended a first time as set forth below in order amend **Appendix D - Benchmarks and Performance** and **Appendix E - Commercial Development Plan**.

NOW, THEREFORE, in consideration of the mutual covenants and promises contained herein, **PHS** and **Licensee**, intending to be bound, hereby mutually agree to the following:

1) The following modifications shall be made to the Agreement:

- a. The following Paragraphs 2.14, 2.15, 2.16, and 6.8 shall be added to the **Agreement**:

2.14 “**Orphan Indication**” means a disease that affects less than two hundred thousand (200,000) people in the United States as defined by the Food and

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Drug Administration or five (5) in ten thousand (10,000) people in the European Union as defined by the European Medicines Agency.

2.15 “**Ultra-Orphan Indication**” means a disease that effects less than one (1) in Fifty Thousand (50,000) people in the United States or the European Union.

2.16 “**Exempt Collaborator**” means a not-for-profit organization or academic institution that has entered a formal collaboration and / or supply agreement with **Licensee** to conduct pre-clinical development and solely sponsor clinical trials of **Licensed Product**, excluding **Supplied Materials**, to treat an **Ultra-Orphan Indication**; in which Licensee may acquire clinical development and data for regulatory approval and sale of a **Licensed Product**.

6.8 Unless otherwise exempted in Article 15, **Licensee** agrees to pay **PHS** benchmark royalties as set forth in Appendix C.

- b. Paragraph 6.3 shall be deleted from the **Agreement** and replaced with the following Paragraph 6.3:

6.3 Unless otherwise exempted in Article 15, **Licensee** agrees to pay **PHS** earned royalties as set forth in Appendix C.

- c. **Appendix D - Benchmarks and Performance of the Agreement** shall be deleted and replaced with the **Appendix D - Benchmarks and Performance** attached to this **First Amendment**.

- d. **Appendix E - Commercial Development Plan** attached to this **First Amendment** shall be added the **Agreement**.

- e. The following Article 15 shall be added to the **Agreement**:

15. EXEMPTION FOR ULTRA-ORPHAN INDICATION RESEARCH

15.1 **Licensee** shall be permitted, upon **PHS** consent, (not to be unreasonably withheld), to manufacture and supply **Licensed Product**, excluding **Supplied Materials**, to an **Exempt Collaborator** for use solely in pre-clinical and clinical development to treat an **Ultra-Orphan Indication**. Prior to commencement of manufacturing of **Licensed Product** for an **Exempt Collaborator**, **Licensee** shall request permission in writing and must obtain written consent from **PHS**. Additional documentation to establish an **Exempt Collaborator** may be required by **PHS**.

For avoidance of doubt, **Licensee** shall retain **Supplied Materials** and shall not release **Supplied Materials** to an **Exempt Collaborator**.

15.2 Upon receipt of written consent from **PHS** for manufacturing of a **Licensed Product** for an **Exempt Collaborator**, **Licensee** shall not be

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obligated to pay **Benchmark** royalties which would have been payable under **Appendix C**, Section V for **Benchmarks** triggered by clinical trials solely sponsored by the **Exempt Collaborator** until such time as **Licensee** exercises its option to acquire the clinical development from the **Exempt Collaborator**.

15.3 Upon acquisition of the clinical development from an **Exempt Collaborator**, **Licensee** shall pay **PHS** royalties which become payable from that point onwards in accordance with **Appendix C**, Section V. **Licensee** must inform **PHS** in writing within **[\*\*]** days of **Licensee's** decision to acquire or not acquire clinical development from the **Exempt Collaborator**.

For avoidance of doubt, **PHS** shall consider **Licensee's** sponsorship or co-sponsorship of a clinical trial or regulatory submission for a **Licensed Product** to treat an **Ultra-Orphan Indication** as an acquisition of clinical development from an **Exempt Collaborator**.

15.4 Earned royalty payments on **Net Sales** specified in Appendix C, Section III shall not be applicable to **Licensed Product** manufactured for research and clinical trials conducted by an **Exempt Collaborator** approved by **PHS** per Paragraph 15.1.

In lieu of earned royalty payments, **Licensee** shall pay **PHS** a royalty payment of **[\*\*]** dollars (\$**[\*\*]**) for each collaboration with an **Exempt Collaborator** approved by **PHS**. Such royalty shall be due within **[\*\*]** days of the date of **PHS** written consent per Paragraph 15.1.

In case several **PHS** licenses apply to the same **Licensed Product**, only a single payment of \$**[\*\*]** shall be payable per collaboration.

- 2) Within **[\*\*]** days of the execution of this **First Amendment**, **Licensee** shall pay **PHS** an amendment issue royalty in the sum of **[\*\*]**US Dollars (\$**[\*\*]**), to be sent to the address specified in Attachment 1.
- 3) In the event any provision(s) of the **Agreement** is/are inconsistent with Attachment 1, such provision(s) is/are hereby amended to the extent required to avoid such inconsistency and to give effect to the payment information in such Attachment 1.
- 4) All terms and conditions of the Agreement not herein amended remain binding and in effect.

- 5) The terms and conditions of this **Amendment** shall, at **PHS**' sole option, be considered by **PHS** to be withdrawn from **Licensee**'s consideration and the terms and conditions of this **Amendment**, and the **Amendment** itself to be null and void, unless this **Amendment** is executed by the **Licensee** and a fully executed original is received by **PHS** within [\*\*] days from the date of **PHS** signature found at the Signature Page.
- 6) This **First Amendment** is effective on December 31, 2009 upon execution by all parties.

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**SIGNATURES BEGIN ON NEXT PAGE**

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The Netherlands

Email Address: p.morgan@amtbiopharma.com

Phone: +31(0)20 566 7509

Fax: +31(0)20 566 9272

II. Official and Mailing Address for Financial notices (Licensee's contact person for royalty payments):

Piers Morgan

\_\_\_\_\_  
Name

Chief Financial Officer

\_\_\_\_\_  
Title

Mailing Address:

Meibergdreef 61  
1105 BA Amsterdam  
The Netherlands

Email Address: p.morgan@amtbiopharma.com

Phone: +31(0)20 566 7509

Fax: +31(0)20 566 9272

Any false or misleading statements made, presented, or submitted to the **Government**, including any relevant omissions, under this **Agreement** and during the course of negotiation of this **Agreement** are subject to all applicable civil and criminal statutes including Federal statutes 31 U.S.C. §§3801-3812 (civil liability) and 18 U.S.C. §1001 (criminal liability including fine(s) or imprisonment).

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**APPENDIX D - BENCHMARKS AND PERFORMANCE**

Licensee agrees to the following Benchmarks for its performance under this Agreement and, within [\*\*] days of achieving a Benchmark, shall notify PHS that the Benchmark has been achieved.

**Benchmarks for Lead Licensed Product (AMT-011 also known as Glybera™)**

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**Benchmarks for other Orphan Indication Licensed Products (AMT-021 or equivalent)**

[\*\*]

**Benchmarks for Licensed Products (AMT-090 or equivalent)**

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**APPENDIX E - COMMERCIAL DEVELOPMENT PLAN**

**AMT-021 for Acute Intermittent Porphyria**

Acute intermittent porphyria (AIP) is an autosomal dominant inherited condition caused by mutations in the porphobilinogen deaminase (PBGD) gene. The PBGD gene is located on chromosome 11 q24.1 -24.2 and spread over fifteen exons. The protein encoded by this gene is a rate-limiting enzyme, the PBGD enzyme, in the haem synthetic pathway.

More than 225 mutations of the PBGD gene have been described, all of them associated with loss of catalytic function. The disease shows incomplete penetrance and only 20-50% of persons with one or more of the described mutations exhibit clinical symptoms of the disease. The genetic disorder results in a 50% reduction of PBGD enzymatic activity. This reduction of hepatic PBGD activity leads to an accumulation of toxic metabolites resulting from the blockade within the haem synthesis pathway. Concentrations of haem precursors porphobilinogen (PBG) and delta-aminolevulinic acid (ALA) increase in blood and urine. Lack of haem and/or accumulation of these metabolites are responsible for the acute attacks characteristic of this disease (Kauppinen et al 2005; Herrick and McColl 2005). Currently, there is no treatment available for the disease.

Over the last couple of years we have explored AMT-021 (replication defective recombinant adeno-associated viral vector, AAV, containing the porphobilinogen deaminase gene) for therapeutic intervention in AIP. AMT-021 is an AAV with pseudotype 5 capsid, which expresses the human PBGD gene under the transcriptional control of a liver specific promoter. The therapeutic expression cassette consists of the human PBGD cDNA (codon optimised for human expression) inserted downstream of the liver specific promoter EalbAAT and upstream of a human PBGD polyadenylation sequence.

AMT-021 acts by delivering the PBGD expression cassette directly into hepatocytes. The increase of PBGD enzymatic activity in the liver of AIP patients will provide sufficient enzyme to prevent the accumulation of toxic metabolites and thus, prevent porphyric attacks.

The aim of the project is to bring AAV5-PBDG therapy to patients. AMT has already secured orphan designation for AAV5-PBDG treatment for AIP in Europe. The table below describes the outline development plans, starting from a research batch production, and moving through to primate proof-of-concept, tox batch, pre-observational study, product development, GMP production, Phase I/II clinical trial, Phase II/III clinical trial, all the way to regulatory filing. Please note that the timelines are preliminary only, and that it is the nature of scientific and clinical development that planned timelines may change.

Preliminary Project Plan (Acute Intermittent Porphyria):

Task	Timelines
Research batch	[**]
PoC in non-human primate	[**]
Tox batch	[**]
Toxicology (12 months)	[**]
Pre-observation study	[**]

Clinical batch	[**]
Interventional Phase I/II clinical trial	[**]
2nd Observational study & Phase II/III clinical trial	[**]
File with EMA	[**]
Market Launch	[**]

Project Plan Details:

The aim of this project is to develop a gene therapy product for the treatment of AIP, and to deliver a data package that is suitable for the submission and approval by the European and North American regulatory authorities.

Vector development and manufacturing

To develop a gene therapy for PBGD deficient patients, AAV5-PBDG product was designed to express the human PBGD gene under the control of a liver specific promoter. AAV5-PBDG was produced in insect cells using the recombinant baculovirus method; sufficient amount of material was produced for efficacy studies in mice. Methods to determine the quantity and purity of the rAAV batches were developed. A purification process including chromatography and filtration steps was developed, further optimization and characterization of the scale-up procedure will be performed before a final batch for toxicology, for proof of principle and for clinical trials can be produced.

PoC in pre-clinical models

Because total deficiency of PBGD is lethal in mice, a compound heterozygous mouse (PBGD+/- referred to as AIP mice) with ~35% of normal hepatic PBGD activity, has been developed as an established model to study AIP. This murine model of AIP exhibits, after disease induction with phenobarbital (Pb), the typical biochemical characteristics of human AIP, notably, decreased hepatic PBGD activity, massively increased urinary excretion of haem precursors (ALA and PBG) and decreased motor function.

AIP mice were used to test the AAV5-PBDG product. The therapeutic effect was evaluated three months after a single intravenous administration of AAV5-PBDG. Efficacy of the therapy was demonstrated as the treatment was able to prevent disease induction with Pb. ALA and PBG levels in treated animals was reduced, and motor disturbance induced by Pb treatment, as measured in the Rotarod test, was almost completely abolished. In addition, PBGD enzymatic activity increased in the AAV5-PBDG treated group 10 times over that of the control group.

This initial PoC will be repeated with the final version of the therapeutic vector following the completion of the vector development and manufacturing optimisation. The final PoC will include the following:

- PoC in rodent disease model
- PoC in non-human primates, based on agreed protocol

GLP Toxicology

The aim of this section is to deliver toxicology study report suitable for the submission to the regulatory authority. The work will entail the following:

- Scientific advice from a regulatory body (AEMPS and/or EMA) for safety and toxicology package
- GLP toxicology study in rodents rats or mice, including any required biodistribution studies



- Supportive data for toxicology study in non-human primates
- GLP germline transmission study

Toxicology study design will take into account:

- Identification of potential target organs of biological activity and of potential target organs of toxicity
- Eventual concomitant medication (e.g. immunosuppressants, standard co-medication)
- Environmental risk/shedding
- Analysis of appropriateness of surrogate markers of efficacy/safety
- Any other relevant issues as may be identified

Clinical observational, pre-intervention study/studies

Before entering the interventional clinical study, an observation clinical study will be conducted to provide baseline information on the course of the disease by recording episodes AIP, abdominal pain, hospitalizations, extent of any possible known or unknown to be related to AIP symptomatology, incidence of (adverse) clinical events per year, etc. Sufficient data will be collected to provide a clinical picture to obtain a baseline data and to determine how efficacy will be shown during the interventional clinical trial.

Phase I/II

The clinical phase I/II should include an estimated minimum [\*\*] patients that are administered the gene therapy drug, and are followed up and clinically assessed for at least [\*\*] months following drug administration. The primary aim of the clinical study will be safety and efficacy of the AAV5-PBDG product. The clinical trial will include all biochemical, imaging, clinical and functional assays to assess the disease state and change therein over time, the phenotypic disease variation, as well as the overall clinical and psychosocial or other health status or change therein over time of the individual trial subjects, both before, during and following drug administration.

Phase II/III & Regulatory submission

After successful completion of Phase I/II study a Phase II/III trial will be conducted with the aim of bringing the AIP therapy to market. We estimate that [\*\*] patients in total would be sufficient for regulatory filing of this product, as AIP is an ultra-orphan disease with a very limited patient number world-wide.

AMT-090 for Parkinson’s Disease

Parkinson’s disease (PD) is a progressive neurodegenerative disease, resulting in tremors, stiffness, slowness of movement, and lack of coordination. Patients are faced with a severely

debilitating disease and a serious loss in quality of life. PD is caused by degeneration and death of nerve cells in a specific part of the brain known as the substantia nigra. These cells produce dopamine, a substance necessary for communication between nerve cells involved in the coordination of movement.

PD is the second most common neurodegenerative disease. It usually affects people over 65, with an estimated total of 4.5 million patients worldwide. Due to increasing life expectancy of the general population, the number of patients with PD is expected to double to around 9 million patients between now and 2030.

An ideal therapy for PD would decrease disability and slow down or halt disease progression. Unfortunately, such treatments are not available yet and current therapies are limited to symptomatic treatment only. These include levodopa, dopamine agonists, monoamine oxidase B (MAO-B) inhibitors and anticholinergic agents.

Glial cell line-derived neurotrophic factor (GDNF) was shown to promote the survival and differentiation of dopaminergic neurons. The therapy aims to protect and enhance the function of the dopamine-producing nerve cells in the brain. To date a number of clinical trials have been conducted in which recombinant GDNF protein has been directly delivered to the PD brain, using a delivery pump device implanted into patients’ abdomen. Although the results were inconsistent, due to the difficulty of delivering protein continuously into the brain via an implanted pump, some patients have shown a significant clinical response to the treatment. It is therefore not a question whether this approach works, because it definitely did in some patients, but rather how it can be done more consistently. AAV-GDNF gene therapy treatment would result in continues delivery of GDNF protein into brain, and is therefore likely to result in significant clinical benefit for PD patients.

AMT has recently started preclinical development of AAV-GDNF gene therapy that will introduce the gene coding for GDNF using recombinant adeno associated virus vector (AAV). AAV serotype 5 has been shown to be the serotype of choice for gene delivery into the brain. After successful proof of concept (POC) and toxicology studies in rodents and primates, AMT will start an extensive clinical development.

Preliminary timelines for AMT-090

Task	Timelines
POC rats	[**]
POC non-human primates	[**]
Toxicology study	[**]
IND / INPD (approval for phase I)	[**]
Start phase I	[**]
Start phase II	[**]
Start phase III	[**]
Filing	[**]
Market introduction	[**]

The aim of this project is to develop a gene therapy product for the treatment of Parkinson’s disease, and to deliver a data package that is suitable for the submission and approval by the European and North American regulatory authorities.

Vector development and manufacturing

To develop a gene therapy for Parkinson’s disease, AAV-GDNF product was designed to express the human GDNF and is produced in insect cells using the recombinant baculovirus method. The AAV5-GDNF is based on AMT’s standard manufacturing process, but in addition incorporates recent new technology of the basic process and makes use of an optimized Rep baculovirus construct in the upstream process and an additional chromatography step in the downstream process. This optimisation delivers enhanced quality and robustness of the AAV5-GDNF product. This process is fully scalable and allows for manufacturing of sufficient GMP-compliant product for PD patients.

Characterization of AAV5-GDNF

The AAV5-GDNF was tested in a functional *in vitro* assay in cultured E13.5 rat DRG explants. Vigorous neural outgrowth was observed, indicating that the produced AAV5-GDNF is capable of mediating secretion of biologically functional recombinant GDNF.

In vivo characterization

Subsequently, an *in-vivo* characterisation of the AAV5-GDNF has been conducted. Three different concentrations of AAV5-GDNF were injected unilaterally into the rat striatum. Brains were analyzed for GDNF expression [\*\*] weeks post injection using immunohistochemistry. Resulting data demonstrated that there is a strong, concentration dependent GDNF expression throughout the injected hemisphere.

PoC in pre-clinical models

The produced AAV5-GDNF will be used to show biological activity and efficacy in animal models of Parkinson’s disease. These experiments will be conducted using rat models of Parkinson’s disease (in collaboration with University of Lund, Sweden) as well as non-human primates model of Parkinson’s disease (in collaboration with CEA, Paris, France). In addition to distribution studies, onset and kinetics of GDNF expression, neurochemical measurements (dopamine and dopamine metabolites), immunohistochemistry and behavioural studies will be conducted to test for functional improvement.

GLP Toxicology

The definitive design of the actual studies will be finalized after discussions with relevant agencies. We propose to conduct a [\*\*] months study in mice and in parallel a [\*\*] months study in non-human primates to account for the safety of the drug. The studies will comprise four test groups: 1. Control (vehicle), 2. Low dose (No observed effect level (NOEL) in the proof-of concept studies), 3. Mid-dose (highest dose considered for clinical studies), and 4. High dose (10 times higher than the mid-dose).

The protocol will include the following evaluations:

- Clinical Signs: recorded daily, beginning 7 days prior to surgery
- 
- Food Consumption: recorded daily, beginning 7 days prior to surgery
  - Body Weight: Once pre-surgery, day of surgery, then bi-weekly
  - Clinical Chemistry: Twice a month presurgery, one week post surgery, then monthly
  - Hematology: Twice a month presurgery, one week post surgery, then monthly
  - Coagulation: Twice a month presurgery, one week post surgery, then monthly
  - Antibodies against GDNF or AAV5 in plasma, twice prior to surgery, monthly thereafter.
  - PK - CSF: To determine if there is GDNF in the CSF, twice prior to surgery, monthly thereafter.
  - Neurological Examination: Twice prior to surgery, Day 7 post surgery, monthly thereafter
  - MRI (T1.T2): Once prior to surgery, within three hours post surgery, and within three days prior to necropsy.
  - Pathology
    1. Gross pathology at necropsy
    2. Selected peripheral tissues collected for histopathological analysis by a Board Certified Pathologist
    3. Complete CNS histopathological assessment by a Board Certified Neuropathologist, peer reviewed by another Board Certified Pathologist
- Q-PCR in selected organs in order to assess any biodistribution of the vector DNA to other organs.

Phase I/II

The primary objective of the clinical phase I/II will be to assess the safety and feasibility of intra-putaminal delivery of AAV5-GDNF to patients with PD. Secondary objectives include measuring clinical efficacy and demonstrating improvement in a surrogate marker end point ((18)F-Dopa PET) as proof of concept.

We are proposing a single centre open label trial of striatally delivered AAV5-GDNF in PD employing a dose escalation design to assess the mentioned primary and secondary outcome measures. We anticipate enrolling [\*\*] patients in this study, with an escalating dose group design with [\*\*] patients in each dose group. We will start with the lowest dose and progress in an incremental way to higher doses.

Primary outcome assessments will be performed at [\*\*] post intra-putaminal infusion of AAV5-GDNF. Clinical secondary outcome assessments will be performed at [\*\*] post intra-putaminal infusion of AAV5-GDNF. (18)F-dopa PET secondary outcome assessments will be performed at [\*\*] months and [\*\*] months post intra-putaminal infusion of AAV5-GDNF.

If feasibility and safety is confirmed and, serial PET imaging demonstrates increased (18)F-dopa uptake with a trend towards clinical improvement, we will proceed to phase 2/3 clinical trials.

Phase II/III. Phase III & Regulatory submission

After successful completion of Phase I/II study, two additional clinical trials will be required. The final plans for these trials will be optimised based on the outcome of the Phase I/II study. We estimate [\*\*] patients to be enrolled in the Phase II/III clinical study, and [\*\*] patients to be

enrolled in the pivotal trial, the details however will be established, based on the outcome of the Phase I/II trial.

#### **EXHIBIT A - Request for Benchmark Exemption With Institut Pasteur**

Walenta, Jeffrey (NIH/OD) [E]

From: Tamara Tugal [ttugal@amtbiopharma.com]  
Sent: Tuesday, October 19, 2010 11:36 AM  
To: Walenta, Jeffrey (NIH/OD) [E]  
Cc: Mark Chadwick  
Subject: Manufacture for not-for-profit organisations and academic institutions exemption; L-107-2007 and L-119-207

Dear Jeffrey

It was nice to talk to you the other day and to have the opportunity to discuss with you AMT's plans to participate in the development of products for the treatment of ultra-orphan disorders. We appreciate your openness to help in the development of products for rare disorders that are being developed by not-for-profit organisations. I know that Mark is working with you on a separate amendment to the license, but I would like to take care of this particular point separately.

Sanfilippo Syndrome IIIB (Sanfilippo B) is a lysosomal storage disorder caused by a deficiency of the enzyme  $\alpha$ -N-acetylglucosaminidase (NaGlu), resulting in a severe degenerative pathology of central nervous system. Sanfilippo B patients appear normal at birth but develop hyperactivity, sleep disorders, loss of speech, mental retardation and dementia in early childhood. Patients with Sanfilippo B will die at around 10-15 years of age. No treatment or cure is currently available. However, only estimated 20 children are born annually with the disease in Western Europe. These numbers are far too small to justify commercial investment in the therapy. Institut Pasteur has limited charity funding available for the development of treatment for this disease, and would like AMT to manufacture the clinical material for them. AMT would like to clarify our position in relation to NIH while we manufacture to Pasteur, as we discussed on the phone. Please see below the proposed text for such an amendment, I hope that it will be acceptable. Please note that in addition to the L-107-2007 license, the L-119-207 license is also applicable, and we would like to have the same arrangement under both agreements to allow us to work with not-for-profit organisations.

Note that we have been approached by Universities attempting to develop treatments for similar diseases. Hence we would like to be able to extend the mechanism to additional product in the future.

#### **Proposed Amendment: Manufacture for not-for-profit organisations and academic institutions exemption.**

AMT will be free to manufacture clinical trial material using the licensed technology for not-for-profit organisations and academic institutions without any obligation of payments to NIH. In the first instance, AMT intends to manufacture material for Institut Pasteur for the treatment of Sanfilippo B syndrome. AMT will have an option to license the program from Institut Pasteur and may acquire the program from Institut Pasteur in the future. If AMT acquires the program

from Institut Pasteur, it will, from this point onwards, pay NIH the milestones and royalties as defined in the L-107-2007 (and / or the L-119-207) license. The mechanism will then be applicable to manufacturing of other products for not-for-profit and academic organisations. AMT will notify NIH of any new products that it intends to manufacture under this exemption, prior to the commencement of manufacturing.

#### **Exhibit B - PHS Consent for Benchmark Exemption with Institut Pasteur**



DEPARTMENT OF HEALTH & HUMAN SERVICES

Public Health Service

National Institutes of Health  
Office of Technology Transfer  
6011 Executive Boulevard, Suite 325  
Rockville, Maryland 20852

(301) 435-5378

March 4, 2011

Tamara Tugal  
Business Development Manager  
Amsterdam Molecular Therapeutics  
Mcibergdreef 61  
1105 BA Amsterdam  
The Netherlands  
Phone Number: +31(0)20 566 7509

**Re: Benchmark Exemption for a Proposed Collaboration Between Amsterdam Molecular Therapeutics ('AMT') and Institut Pasteur**

Dear Ms. Tugal:

Thank you for your request dated October 19, 2010 for a benchmark royalty exemption for collaboration agreements with not-for-profit organizations or academic institutions to conduct clinical development of potential treatments for ultra-orphan diseases.

Pending execution of the First Amendment to PHS License Reference No. L-107-2007/0 effective May 2, 2007 (the ‘Agreement’), PHS provides consent per Article 15 for AMT to provide material for use in clinical development of a treatment for lysosomal storage disorder Sanfilippo Syndrome IIIB at the Institut Pasteur,

Congratulations on your new collaboration. When AMT makes a decision about acquisition of clinical development from Institut Pasteur, please inform PHS as soon as possible.

We appreciate AMT’s continued interest in PHS technologies. If you have any questions, please do not hesitate to contact our office at any time

Sincerely,

/s/ Richard U. Rodriguez

Richard U. Rodriguez

Director, Division of Technology Development & Transfer

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### **ATTACHMENT 1 - ROYALTY PAYMENT OPTIONS**

The OTT License Number **MUST** appear on payments, reports and correspondence.

#### **Automated Clearing House (ACH) for payments through U.S. banks only**

The NTH encourages our licensees to submit electronic funds transfer payments through the Automated Clearing House (ACH). Submit your ACH payment through the U.S. Treasury web site located at: **https://www.pay.gov**. Locate the “NIH Agency Form” through the Pay.gov “Agency List”.

#### **Electronic Funds Wire Transfers**

The following account information is provided for wire payments. In order to process payment via Electronic Funds Wire Transfer sender **MUST** supply the following information within the transmission:

Drawn on a **U.S. bank account** via FEDWIRE should be sent directly to the following account;

Beneficiary Account:	Federal Reserve Bank of New York or TREAS NYC
Bank:	Federal Reserve Bank of New York
ABA#	021030004
Account Number:	75080031
Bank Address:	33 Liberty Street, New York, NY 10045
Payment Details:	License Number (L-XXX-XXXX)
	Name of Licensee

Drawn on a **foreign bank account** should be sent directly to the following account. Payment must be sent in **U.S. Dollars (USD)** using the following instructions:

Beneficiary Account:	Federal Reserve Bank of New York/ITS or FRBNY/ITS
Bank:	Citibank N.A. (New York)
SWIFT Code#	CITIUS33
Account Number:	36838868
Bank Address:	388 Greenwich Street, New York, NY 10013
Payment Details: (Line 70):	NIH 75080031
	License Number (L-XXX-XXXX)
	Name of Licensee
Detail of Charges (line 71a):	Charge Our

#### **Checks**

All checks should be made payable to “NIH Patent Licensing”

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Checks drawn on a **U.S. bank account** and sent by US Postal Service should be sent directly to the following address:

National Institutes of Health (NIH)  
P.O. Box 979071  
St. Louis, MO 63197-9000

Checks drawn on a U.S. bank account and sent by **overnight or courier** should be sent to the following address:

US Bank  
Government Lockbox SL-MO-C2GL  
1005 Convention Plaza

Checks drawn on a **foreign bank account** should be sent directly to the following address:

National Institutes of Health (NIH)  
Office of Technology Transfer  
Royalties Administration Unit  
6011 Executive Boulevard  
Suite 325, MSC 7660  
Rockville, Maryland 20852

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NATIONAL INSTITUTES OF HEALTH

**SECOND AMENDMENT TO L-107-2007/0**

This is the second amendment (“**Second Amendment**”) of the agreement by and between the National Institutes of Health (“**NIH**”) within the Department of Health and Human Services (“**HHS**”), and UniQure biopharma B.V. (formerly Amsterdam Molecular Therapeutics N.V. (AMT)) having an effective date of May 2, 2007 and **NIH** Reference Number L-107-2007/0, and having been amended for the first time on December 31, 2009 (**NIH** Reference L-107-2007/1) (“**Agreement**”). This **Second Amendment**, having **NIH** Reference Number L-107-2007/2, is made between the **NIH** through the Office of Technology Transfer, **NIH**, having an address at (6011 Executive Boulevard, Suite 325, Rockville, Maryland 20852-3804, U.S.A., and UniQure biopharma B.V. (formerly Amsterdam Molecular Therapeutics N.V. (AMT)), having an office at Meibergdreef 61, 1105 BA Amsterdam, The Netherlands (“**Licensee**”). This **Second Amendment** includes, in addition to the amendments made below, 1) a Signature Page, and 2) Attachment 1 (Royalty Payment Information).

WHEREAS, **NIH** and **Licensee** desire that the **Agreement** be amended a second time as set forth below in order to

- a) Change the name of **Licensee** from Amsterdam Molecular Therapeutics N.V. (AMT) to UniQure biopharma B.V. (UniQure). This name change is the result of a transaction that took place on 30 March 2012, whereby AMT, a public company, was liquidated and all its operations and stocks were transferred to UniQure, a privately held company.
- b) Modify language related to financial terms associated with sublicensing, so as to cause a reduction in financial obligations due to **NIH** from sublicensing of the **Agreement** by **Licensee** in order to expedite the development of therapeutics for rare diseases.

NOW, THEREFORE, in consideration of the mutual covenants and promises contained herein, **NIH** and **Licensee**, intending to be bound, hereby mutually agree to the following:

- 1)
  - a) In Cover page following the list of “licensed patent and patent application”, the name of **Licensee** has been changed to UniQure biopharma B.V.
  - b) In the signature page under “signature of authorized official”, under “Official and Mailing Address for **Agreement** notices”, and under “Official and Mailing Address for Financial notices” “Amsterdam Molecular Therapeutics” has been changed to UniQure biopharma B.V.
  - c) In the caption of the **Agreement** AMT is changed to UniQure.
- 2)
  - a) Paragraph 6.6 is deleted in its entirety and replaced with the following:

6.6 No multiple royalties shall be payable if any **Licensed Products** or **Licensed Processes** are covered by more than one of the **Licensed Patent Rights**. In the event that this **Agreement** and **NIH** license L-116-2011/0 as amended from time to time apply to the same product sold by the **Licensee** or its sublicensees, then the **Licensee** shall only pay earned

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royalties, benchmark royalties, and sublicensing royalties under **NIH** license L-116-2011/0.

- b) In Appendix C, the second occurrence of Roman numeral “IV” at the bottom of the page is replaced with Roman numeral “VI”. Section VI has been deleted in its entirety and replaced with the following:

**Licensee** agrees to pay **NIH** additional sublicensing royalties on the fair market value of any consideration received for granting each sublicense within [\*\*] days of the execution of each sublicense as follows:

- (i) For any sublicense executed by the **Licensee** before the [\*\*], **Licensee** agrees to pay a sublicensing royalty of [\*\*] percent ([\*\*] %); and
- (ii) For any sublicense executed by the **Licensee** after the [\*\*], **Licensee** agrees to pay a sublicensing royalty of [\*\*] percent ([\*\*] %); and
- (iii) For any sublicense executed by the **Licensee** either [\*\*], **Licensee** agrees to pay a sublicensing royalty of [\*\*] percent ([\*\*] %).

Contractual payments made by a sublicensee to the **Licensee** or an **Affiliate** received after the effective date of this **Agreement** for costs, services and expenses for the **Licensee** or **Affiliate** to perform research and development activities, or to conduct, supervise or participate in one or more clinical trial(s) for the development of the **Licensed Products**, or to manufacture clinical and commercial batches of **Licensed Products**, shall not be accounted for in the calculation of sublicensing royalties.

- 3) **Licensee** shall pay **NIH** an amendment issue royalty in the sum of [\*\*] US Dollars (\$[\*\*]) as follows:

- i) [\*\*] Dollars (\$[\*\*]) shall be paid by **Licensee** within [\*\*] days of the effective date of this **Second Amendment**.

ii) The remaining amount of [\*\*] Dollars (\$[\*\*]) shall be paid to **NIH** upon execution by **Licensee** of any new sublicensing or partnership agreement, or on the first anniversary of this **Second Amendment**, whichever occurs first.

- 4) In the event any provision(s) of the **Agreement** is/are inconsistent with Attachment 1, such provision(s) is/are hereby amended to the extent required to avoid such inconsistency and to give effect to payment information in such Attachment 1.
- 5) All terms and conditions of the **Agreement** not herein amended remain binding and in effect.
- 6) The terms and conditions of this **Second Amendment** shall, at **NIH**' sole option, be considered by **NIH** to be withdrawn from **Licensee's** consideration and the terms and conditions of this **Second Amendment**, and

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the **Second Amendment** itself, to be null and void, unless this **Second Amendment** is executed by **Licensee** and a fully executed original is received by **NIH** within [\*\*] days from the date of **NIH** signature found at the Signature Page.

- 7) This **Second Amendment** is effective on May 31, 2013 upon execution by all parties.
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## SECOND AMENDMENT TO L-107-2007/0

### SIGNATURE PAGE

In Witness Whereof, the parties have executed this **Second Amendment** on the dates set forth below. Any communication or notice to be given shall be forwarded to the respective addresses listed below.

For **NIH**:

/s/ Richard U. Rodriguez  
Richard U. Rodriguez  
Director, Division of Technology Development and Transfer  
Office of Technology Transfer  
National Institutes of Health

5-23-13  
Date

Mailing Address or E-mail Address for Agreement notices and reports:

Chief, Monitoring & Enforcement Branch, DTD  
Office of Technology Transfer  
National Institutes of Health  
6011 Executive Boulevard, Suite 325  
Rockville, Maryland 20852-3804 U.S.A.

E-mail: LicenseNotices\_Reports@mail.nih.gov

For **Licensee** (Upon information and belief, the undersigned expressly certifies or affirms that the contents of any statements of **Licensee** made or referred to in this document are truthful and accurate.):

/s/ Jorn Alday  
Jorn Alday, CEO, uniQure biopharma B.V.

5-31-13  
Date

- I. Official and Mailing Address for **Agreement** notices:  
Chief Executive Officer;  
Legal@uniqure.com
- II. For invoices, payments, and Financial notices (including royalty payments):  
Finance Dept  
Finance@uniqure.com

uniQure biopharma B.V.  
Meibergdreef 61  
1105BA Amsterdam  
The Netherlands

Phone: 0031 205667394  
Fax: 0031 20 566 9272

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Any false or misleading statements made, presented, or submitted to the **Government**, including any relevant omissions, under this **Agreement** and during the course of negotiation of this **Agreement** are subject to all applicable civil and criminal statutes including Federal statutes 31 U.S.C. §§3801-3812 (civil liability) and 18 U.S.C. §1001 (criminal liability including fine(s) or imprisonment).

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## ATTACHMENT 1 - ROYALTY PAYMENT OPTIONS

The OTT License Number MUST appear on payments, reports and correspondence.

### Automated Clearing House (ACH) for payments through U.S. banks only

The NIH encourages our licensees to submit electronic funds transfer payments through the Automated Clearing House (ACH). Submit your ACH payment through the U.S. Treasury web site located at: <https://www.pay.gov>. Locate the "NIH Agency Form" through the Pay.gov "Agency List".

### Electronic Funds Wire Transfers

The following account information is provided for wire payments. In order to process payment via Electronic Funds Wire Transfer sender MUST supply the following information within the transmission:

Drawn on a **U.S. bank account** via FEDWIRE should be sent directly to the following account:

Beneficiary Account:	Federal Reserve Bank of New York or TREAS NYC
Bank:	Federal Reserve Bank of New York
ABA#	021030004
Account Number:	75080031
Bank Address:	33 Liberty Street, New York, NY 10045
Payment Details:	License Number (L-XXX-XXXX)
	Name of Licensee

Drawn on a **foreign bank account** should be sent directly to the following account. Payment must be sent in **U.S. Dollars (USD)** using the following instructions:

Beneficiary Account:	Federal Reserve Bank of New York/ITS or FRBNY/ITS
Bank:	Citibank N.A. (New York)
SWIFT Code:	CITIUS33
Account Number:	36838868

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Bank Address:	388 Greenwich Street, New York, NY 10013
Payment Details (Line 70):	NIH 75080031 License Number (L-XXX-XXXX)
	Name of Licensee
Detail of Charges (line 71 a):	Charge Our

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### Checks

All checks should be made payable to "NIH Patent Licensing"

Checks drawn on a **U.S. bank account** and sent by US Postal Service should be sent directly to the following address:

National Institutes of Health (NIH)  
P.O. Box 979071  
St. Louis, MO 63197-9000

Checks drawn on a U.S. bank account and sent by **overnight or courier** should be sent to the following address:

US Bank  
Government Lockbox SL-MO-C2GL  
1005 Convention Plaza  
St. Louis, MO 63101  
Phone: 314-418-4087

Checks drawn on a **foreign bank account** should be sent directly to the following address:

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NATIONAL INSTITUTES OF HEALTH

THIRD AMENDMENT TO L-107-2007/0

This is the third amendment (“**Third Amendment**”) of the agreement by and between the National Institutes of Health (“**NIH**”) within the Department of Health and Human Services (“**HHS**”), and UniQure biopharma B.V. (formerly Amsterdam Molecular Therapeutics (AMT) B.V.) having an effective date of May 2, 2007 as amended for the first time on December 31, 2009, and amended for the second time on May 31, 2013, and having **NIH** Reference Number L-107-2007/0, L-107-2007/1, and L-107-2007/2 respectively (“**Agreement**”). This **Third Amendment**, having **NIH** Reference Number L-107-2007/3, is made between the **NIH** through the Office of Technology Transfer, **NIH**, having an address at 6011 Executive Boulevard, Suite 325, Rockville, Maryland 20852-3804, U.S.A., and UniQure biopharma B.V. (formerly Amsterdam Molecular Therapeutics (AMT) B.V.), having an office at Meibergdreef 61, 1105 BA Amsterdam, The Netherlands (“**Licensee**”). This **Third Amendment** includes, in addition to the amendments made below, a Signature Page.

WHEREAS, **NIH** and **Licensee** desire that the **Agreement** be amended a third time as set forth below in order to a) clarify the **Field of Use**, and b) to update appendices D and E of the **Agreement** to capture all of **Licensee**’s past, current and future **Commercial Development Plan**,

NOW, THEREFORE, in consideration of the mutual covenants and promises contained herein, **NIH** and **Licensee**, intending to be bound, hereby mutually agree to the following:

- 1) In Appendix B Paragraph I, replace the **Licensed Field of Use** with the following:  
  
Use of **Licensed Patent Rights** for development and sale of AAV related products
- 2) Replace Appendix D with Appendix D attached to this **Second Amendment** as EXHIBIT 1.
- 3) Replace Appendix E with Appendix E attached to this **Second Amendment** as EXHIBIT 2.
- 4) All terms and conditions of the **Agreement** not herein amended remain binding and in effect.
- 5) The terms and conditions of this **Third Amendment** shall, at **NIH** sole option, be considered by **NIH** to be withdrawn from **Licensee**’s consideration and the terms and conditions of this **Third Amendment**, and the **Third Amendment** itself, to be null and void, unless this **Third Amendment** is executed by **Licensee** and a fully executed original is received by **NIH** within [\*\*] days from the date of **NIH** signature found at the Signature Page.
- 6) This **Third Amendment** is effective on the date of execution by the last party to execute this **Third Amendment**.

A-041-2014

**CONFIDENTIAL**

second Amendment of L-107-2007/0  
Model 09-2006 (updated 8-2010)

[Final] UniQure biopharma, B.V.

October 29, 2013  
L-107-2007/3

THIRD AMENDMENT TO L-107-2007/0

SIGNATURE PAGE

In Witness Whereof, the parties have executed this **Third Amendment** on the dates set forth below. Any communication or notice to be given shall be forwarded to the respective addresses listed below.

For **NIH**:

/s/ Ricahrd U. Rodriguez  
Richard U. Rodriguez  
Director, Division of Technology Development and Transfer  
Office of Technology Transfer  
National Institutes of Health

11-6-13  
Date

Mailing Address or E-mail Address for **Agreement** notices and reports:

Chief, Monitoring & Enforcement Branch, DTD  
Office of Technology Transfer  
National Institutes of Health  
6011 Executive Boulevard, Suite 325  
Rockville, Maryland 20852-3804 U.S.A.

E-mail: LicenseNotices\_Reports@mail.nih.gov



For **Licensee** (Upon information and belief, the undersigned expressly certifies or affirms that the contents of any statements of **Licensee** made or referred to in this document are truthful and accurate.):

/s/ Piers J. Morgan — PJ Morgan CFO

Piers J Morgan, CFO, uniQure biopharma B.V.

November 11, 2013

Date

- I Official and Mailing Address for **Agreement** notices:  
Chief Executive Officer;  
Legal@uniqure.com
- II For invoices, payments, and Financial notices (including royalty payments):  
Finance Dept  
Finance@uniqure.com

uniQure biopharma B.V.  
Meibergdreef 61  
1105BA Amsterdam  
The Netherlands

Phone:0031 205667394

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Fax:0031 20 566 9272

Any false or misleading statements made, presented, or submitted to the **Government**, including any relevant omissions, under this **Agreement** and during the course of negotiation of this **Agreement** are subject to all applicable civil and criminal statutes including Federal statutes 31 U.S.C. §§3801-3812 (civil liability) and 18 U.S.C. §1001 (criminal liability including fine(s) or imprisonment).

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EXHIBIT 1

APPENDIX D — BENCHMARKS AND PERFORMANCE (L-107/2007)

**Licensee** agrees to the following Benchmarks for its performance under this **Agreement** and, within **[\*\*]** days after achieving a **Benchmark**, shall notify **PHS** that the **Benchmark** has been achieved.

Note: No formal Phase III clinical trial is required for **Marketing Approval** for any **Orphan Indication**

**Benchmarks** for lead **Licensed Product** (AMT-011 also known as Glybera™).

[\*\*]

**Benchmarks** for another **Orphan Indication Licensed Product** (AMT-021 or equivalent)

[\*\*]

**Benchmarks** for a non-**Orphan Indication Licensed Product** (AMT-090 or equivalent)

[\*\*]

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EXHIBIT 2

APPENDIX E — COMMERCIAL DEVELOPMENT PLAN (L-107-2007)

The table below (table 1) presents a comprehensive list of all uniQure research and development projects utilizing the Licensed Patent Rights, according to main disease site and divided into projects that are, **a)** commercial projects, **b)** already in development stages, **c)** active research (there is already internal research activity ongoing and **d)** exploratory research projects (currently being considered as potential projects worth further investigation in the near future).

Table 1: uniQure R&D projects

Commercial Projects	Brain & CNS (AAV5 based unless otherwise indicated)	
	[**]	[**]
Development Projects	[**]	[**]
Active Research Projects	[**]	[**]
Exploratory Research Projects	[**]	[**]

Detailed information on the commercial, development and active research projects is provided below.

**NOTE: All dates contained in this Commercial Development Plan are projected estimates only.**

## **A) Commercial Projects**

### **Glybera**

Glybera, an AAV1 based product for lipoprotein lipase deficiency, was approved under exceptional circumstances by the EMA in October 2012. Currently uniQure is in the phase of preparation of product launch, for which it has found a partner in Chiesi. It is our aim together with Chiesi to launch in Europe in the first half of 2014. The commercialization agreement with Chiesi has been shared with NIH.

uniQure is actively working on post-approval commitments which came with the approval under exceptional circumstances. Besides some CMC related commitment, there is a commitment for a Phase IV study to collect more biomarker data on the chylomicron handling before and after treatment with Glybera as well as setting up a registry for LPLD patients. Both studies are in progress and in accordance with timelines of the commitments to the EMA.

Furthermore, uniQure has initiated the first campaign for the product of commercial product. This campaign is ongoing and will result in release of the first batch in quarter 1, meeting the planning of the launch of Glybera.

In the context of the European pricing and reimbursement (P&MA) preparations for Glybera, these have been initiated and efforts are ongoing to prepare a pricing strategy as well as a European Core value Dossier (CVD). The agency selected to support the development of the pricing strategy as well as the European CVD has been nominated following a thorough screening and selection process, - the mutual CDA does not allow a disclosure of the agency, which has been agreed with our partner Chiesi. Please be aware that the continued management of the P&MA preparations is now led by Chiesi, who have the commercialization rights for Glybera in the EU.

The P&MA dossier is the crystallization of a multifactorial approach to P&MA and will focus on the Clinical and Epidemiology Data & Publications, Economic Tools & Data, P&MA Strategy and Goals as well as P&MA

### **Implementation & Tactics.**

The Initial evaluation and assessment of the current available market information is sparse due to the new concept of introducing the first gene therapy, a market which today has basically no treatment for LPLD patients and the first time introduction of a one-time administration treatment for an ultra-rare disease.

Key clinical data will be incorporated into the CVD and translated into value statements and economic benefits that will provide rationale and evidence for the positioning of Glybera® with key stakeholders, especially physicians and payers.

The Core Value Dossier is, therefore, a crucial vehicle to deliver the P&MA strategy for Glybera® and support tactical execution. Based on the initial preparations, a stepwise approach to the design and development of the Core Value Dossier for Glybera® has been planned.

In the event that serious gaps exist in the data and/or major problems are identified in this initial review, with regard to the usability and credibility of the data with payers and customers, these issues will be discussed between Chiesi and uniQure.

The Glybera® Core Value Dossier will be the internal reference document summarizing the available evidence and documentation and developing these into value statements and approaches to the various relevant stakeholders.

The CVD structure is designed to bring consistency to the messages delivered in each country and, at the same time, provide the depth of information on each point to allow for the essential local market tailoring.

As per 30th September 2013, Chiesi has reported having one informal meeting with a European Health authority to discuss the process and management of the P&MA dossier. As the meeting was informal there was no agenda and no official minutes have been taken. As per 30th September, no reimbursement reports are available. The on-going development of the CVD and the pricing strategy are managed by the same agency.

Discussions with the FDA were started in August of 2013, which will be followed by filing of an IND in Q1 of 2014.

uniQure is currently developing a geographical expansion strategy to find partners and product approval in other territories, such as Israel, Canada and South Korea.

## **Liver Programs**

### **B) Development Programs**

1. AMT-021 for Acute Intermittent Porphyria

#### ***Disease Background***

Acute Intermittent Porphyria, or AIP, is a rare liver metabolic disorder resulting from mutations in the PBGD gene. This gene encodes for the enzyme porphobilinogen deaminase (also known as hydroxymethylbilane synthase — HMBS), a liver protein necessary for the production of heme, a component of hemoglobin and other blood proteins. Insufficient activity of this protein leads to an accumulation of toxic metabolites (ALA and PBG), resulting in a wide variety of serious clinical problems, including acute, severe abdominal pain, muscular weakness and an array of neurologic manifestations, including psychiatric episodes, seizures and coma. In the majority of cases, attacks are triggered by precipitating factors such as hormonal fluctuations, infections, drugs and dietary changes.

Long-term consequences may include irreversible nerve damage, liver cancer and kidney failure. Patients with AIP experience regular hospitalizations and extremely poor quality of life, and may in some cases require liver transplants. Acute attacks can be life-threatening. Current therapies only target the disease symptoms and do not prevent attacks or fully minimize or control their consequences.

A recent epidemiological study reported that, in Europe (excluding Sweden), the incidence of AIP is 0.13 per million population per year and based on that they estimated a prevalence of 5.9 per million population (Elder et al., 2012). In Sweden the incidence and prevalence of AIP are about four times higher than in the rest of Europe due to a founder effect originating in Lapland (Floderus et al., 2002). The frequency in the United States is estimated to be 1-5 cases per 100,000 population (www.emedicine.medscape.com/article/205220-overview#a0199).

Overview of AMT-021 Program

The goal of our AMT-021 program is to provide long-term normalization of the PBGD protein in order to prevent acute AIP attacks and their complications.

The program has been developed through a collaborative agreement with the Foundation for Applied Medical Research (FIMA), its Center for Applied Medical Research (CIMA) and its commercialization arm, DIGNA Biotech, of the University of Navarra (Pamplona, Spain). Part of the funding to support for the Phase I trial (including GLP safety & toxicology studies and the observational trial) was secured through the European Commission Framework Programme 7 award (€3.3 million, grant agreement 261506) made to the AIPGENE consortium (www.aipgene.org/), of which uniQure is a partner.

UniQure holds an exclusive license to the gene cassette being used in the Phase I clinical trial. Under our agreement with DIGNA Biotech and the other consortium members, **Licensee** has an exclusive right to all data related to the program.

Preclinical Development

- Product Profile

AMT-021 is designed to be delivered systemically through a peripheral vein in a single administration.

AMT-021 or rAAV5-hPBGD, is a recombinant adeno-associated vector of serotype 5, consisting of:

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- Inverted terminal regions or ITRs of the adeno-associated serotype 2
- A human codon optimized porphobilinogen deaminase gene or hPBGDco as the therapeutic gene
- A liver specific promoter constituted by the albumin enhancer (Ealb) and the alfa-1-antitrypsin promoter (hAAT)

- Pre-clinical Proof of Concept

Pre-clinical proof of concept (PoC) studies have been performed using the AIP murine model developed by Lindberg et al. (1999). In these studies, long term therapeutic efficacy was achieved. More specifically, at 5x10<sup>13</sup> gc/kg, metabolic correction of the hepatic PBGD enzyme activity, normalization of the PBG and ALA precursor’s accumulation in urine and improvement of the motor coordination were observed. Additionally, a complete neurological study indicated the correction of neurotoxic porphyrin precursors was able to restore nerve conduction and the impaired peripheral neuropathy.

In non-human primates (NHP) treated with AMT-021 at a dose of 5x10<sup>13</sup> gc/kg endogenous PBGD enzymatic activity increased by a factor of two in male and between three and five times in female animals.

- Non-clinical safety & toxicology studies

The following table presents a summary of the AMT-021 non-clinical safety and toxicology studies that have been conducted to support the clinical development program.

Parameter to be assessed	Study performed	Results
[**]	[**]	[**]
[**]	[**]	[**]

4

[**]	[**]	[**]
[**]	[**]	[**]
[**]	[**]	[**]
[**]	[**]	[**]

1.1.1 Summary of AMT-021 Preclinical Development Program

Single intravenous administration of AMT-021 into wild type mice and Rhesus macaques results in:

- Efficient liver transduction resulting in dose dependent increase in viral RNA copy numbers and in turn producing increased PBGD activity
- No morbidity, no changes in body weight or food intake
- No changes in biochemistry, hematology, coagulation and urinalysis associated with AAV5-hPBGD
- Negative vector shedding [\*\*] days after viral administration in serum, saliva, nasal secretions, urine, faeces and semen
- Tissue biodistribution that is mainly limited to liver although some significant transduction was detected in spleen, lymph nodes, heart and adrenal glands

- **Specific hepatic PBGD expression**

#### Clinical Development Program

The key regulatory and clinical development best estimate milestones for AMT-021 include the following,

- EMA Orphan Drug Designation (EU/3/09/632) [\*\*]
- FIMA/ CITA/ UTE/ DIGNA - AMT Collaborative Agreement [\*\*]
- EU-FP7 AIPGene Consortium [\*\*]
- Observational Study AEMPS approval [\*\*]

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- Observational Study start [\*\*]
- Phase I Study AEMPS approval [\*\*]
- Phase I Study: first patient treated [\*\*]
- Phase I Study: last patient treated [\*\*]

#### Expected milestones

- Phase II/III start: [\*\*]
- MAA/ NDA submission: [\*\*]
- Observational trial

A prospective non-interventional (pre-treatment) observational study started at the end of 2011 that aims to assess the evolution of disease-related clinical and laboratory parameters in time, as well as characterize aspects of disease management such as AIP-related hospitalization. This baseline assessment is intended to study possible relationships between biochemical parameters and clinical *endpoints that* will in turn be valuable in evaluating any signs of efficacy in the Phase I trial as well as in subsequent trials. [\*\*] patients are expected to be enrolled who after completion of this observational phase would then enter the interventional stage of the program, i.e., first-in-human clinical study (Phase I). The observational study is to last for at least [\*\*] months for each participant.

To date all [\*\*] AIP-patients have been recruited into the observational study and all but one have completed a minimum of [\*\*] months pre-treatment assessments. The last patient completed the observational study in August 2013.

- Phase I trial

The Investigational Medicinal Product Dossier (IMPD) was submitted to the AEMPS (Spanish Agency for Medicines and Medical Devices) in June 2012 and was approved by the Agency in October 2012.

The Phase I study is a multicenter, open label, prospective, interventional, single dose, dose-escalation clinical trial to investigate the safety and tolerability of AAV5-hPBGDCo (AMT-021) in patients with severe Acute Intermittent Prophyria (Eudra CT no. 2011-005590-23).

The primary objective is to assess the safety of systemic administration and determine the maximum tolerated doses (MTD). Secondary objectives include urinary levels of toxic metabolites (ALA and PBG), disease symptoms evaluation, quality of life evaluation and assessment of pharmacokinetics. Exploratory objectives include, neurological involvement, identification of novel biomarkers and pharmacokinetic modeling.

The Phase I study was initiated in December 2012 in the Department of Medicine (Liver Unit) at the University Clinic of the University of Navarra (Pamplona, Spain). There are [\*\*] patients per cohort and [\*\*] cohorts in the trial (each cohort receiving  $5 \times 10^{11}$ ,  $2 \times 10^{12}$ ,  $6 \times 10^{12}$  or  $1.8 \times 10^{13}$  gc/kg) and all patients will be followed- up for [\*\*] as part of the Phase I study.

All [\*\*] patients who completed the observational trial have also been treated as part of the Phase I study. In the [\*\*] treated patients, no Serious Adverse Events, Treatment Emergent Adverse Events or Liver Events (Dose Limiting Toxicities - DLT's) related to the study medication have been observed to date.

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- Future Clinical Development

It is envisaged that the Phase II/III will be a confirmatory trial where the study population and the outcomes to be assessed (efficacy endpoints — clinical and biochemical) will be based on those as for Phase I. **Licensee** also intends to carry out the study in both Europe and the USA.

#### Summary of AMT-021 Clinical Development Program

- **The first time an AAV5 gene therapy product has been tested in humans**
- **The first time an AAV gene therapy product has been tested in humans at such high dose, i.e.,  $1.8 \times 10^{13}$  gc/kg**

- **No Serious Adverse Events, Treatment Emergent Adverse Events or Liver Events (DLT's) related to the study medication have been observed in the Phase I study to date**
  - **The Phase I is expected to be completed in [\*\*] and Phase II/III is expected to start by the end of [\*\*]**
  - **The Phase II/III program will run in parallel in Europe and US where MAA and NDA, respectively, are expected in [\*\*]**
2. AMT-060 for Hemophilia B

## Disease Background

Hemophilia B is a serious inherited orphan disease in males characterized by insufficient blood clotting. The condition can lead to repeated and sometimes life-threatening episodes of external and internal bleeding following accidental trauma or medical interventions. The episodes may cause long-term damage, for example to the joints, and may be fatal if they occur in the brain. The deficient blood clotting is caused by the lack of functional human Factor IX, or hFIX, a blood clotting factor, as a result of mutations in the gene responsible for encoding this essential protein. The presence of hFIX at greater than 1% of normal levels has a therapeutic effect in promoting clotting. The current standard treatment is prophylactic protein replacement therapy, in which frequent intravenous administrations of recombinant Factor IX (often 2-3 times per week) are required to stop or prevent bleeding. Protein replacement therapy is costly (\$150,000-200,000 per patient per year) and burdensome, and does not completely prevent bleeding.

The total Hemophilia B patient population in the European Union and the United States is estimated at approximately 25,000, according to the World Federation of Hemophilia 2010 Report on the Annual Global Survey. About 40% of individuals with the disease have a severe disorder, characterized by functional factor IX levels that are less than 1% of normal, whereas moderately severe Hemophiliacs (about 30% of the Hemophiliac population) have 1%-5% of normal and those with the mild phenotype (the remaining 30%) have between 5% and 40% of normal factor IX levels (www.orpha.net). Based on these estimates **Licensee** believes that approximately 70-85% of the worldwide patient population would be eligible for treatment with gene therapy. **Licensee** believes that the treatment would not be appropriate for those patients with very mild disease phenotype.

## Overview of AMT-060 Program

The goal of our AMT-060 program is to restore blood clotting on a long-term basis through the introduction of the functional gene for hFIX into the patient's liver cells. **Licensee** is currently in the process of finalizing pivotal (GLP) safety and toxicology studies and preparing to conduct a Phase I trial.

### *Preclinical Development*

- Product Profile

AMT-060 is designed to be delivered systemically through a peripheral vein in a single administration.

The use of recombinant adeno-associate vectors (rAAV) of serotype 5 (rAAV5) for targeted gene delivery to the liver was pioneered by St. Jude Children's Research Hospital (SJCRH) where for pre-clinical experiments the hFIX expression cassette was packaged into AAV5 capsids in HEK-293T mammalian cells. HEK-293 produced AAV5-hFIX is not suitable for further development because as a production system it is not amenable to large-scale production. To allow up scaling, the expression cassette has now been transferred into uniQure's proprietary baculovirus expression vector system (BEVS) that can be adapted to a GMP setting. The resulting vector produced using the baculovirus expression system is termed AAV5-hFIXco or AMT-060. **Licensee** also holds a license from SJCRH to the gene cassette used in the currently ongoing Phase I/II AAV 2/8-LP1-hFIXco trial.

AMT-060, rAAV5-hFIXco, is a recombinant adeno-associated vector of serotype 5, consisting of:

- Inverted terminal regions (or ITRs) of the adeno-associated serotype 2
- A human codon optimized FIX gene (or hFIXco) as the therapeutic gene
- The liver specific promoter, LP1, derived from the human apolipoprotein hepatic control region and the human alpha-1-antitrypsin (or hAAT) promoter
- *Virus serotype selection*

The hFIXco expression cassette and rAAV5 or AAV8 vectors have been extensively studied in mice and non-human primate. Both vectors have been shown to have similar tropism to (preference to transduce) the liver (Nathwani et al., 2007) and AAV5-hFIXco was shown to mediate expression of significant levels of human factor IX in non-human primates (NHP) during a monitoring period of more than 5 years (Nathwani et al., 2011). In this study none of the animals presented elevated liver enzymes levels or other signs of toxicity during the whole observation period. Liver examination by MRI scanning did not reveal any abnormalities in any of the animals.

These pre-clinical data suggest that i.v. administration of the AAV5-hFIXco vector is able to mediate a similar level of human factor IX as presented for AAV8-hFIXco, and such administration is not associated with safety concerns or immunogenicity against the human factor IX.

- *Pre-clinical Proof of Concept*

Pre-clinical PoC studies have been carried out in wild type mice, non-human primates (NHP) and are currently being completed in transgenic Hemophilia B mice. In wild type mice (C57BL/6) intravenous administration of AMT-060 mice resulted in dose-dependent levels of (human) factor IX levels in murine plasma as determined by ELISA. Human factor IX levels amounted up to 11% of those in normal human plasma 4 weeks after infusion of  $5 \times 10^{12}$  gc/kg, demonstrating that AAV5-hFIXco produced in the BEVS is biologically active.

In Rhesus monkeys dosed with AMT-060 (5x10<sup>12</sup> gc/kg) by intravenous infusion, human FIX levels peaked to 7%-16% of normal human levels one week after infusion, and stabilized to 5-10% of normal human levels 4 weeks after infusion until sacrifice (12 weeks after dosing). These kinetics are in accordance with those observed in previous studies (Nathwani et al., 2007; Jiang et al., 2006), indicating that i.v. administration of AAV5-hFIXco produced in BEVS results in a level of factor IX in plasma that is similar to that produced using AAV5-hFIXco produced in HEK293 cells. Post mortem, (RT)-QPCR demonstrated homogeneous vector DNA delivery and transgene expression in the liver. No signs of adverse reactions were observed. Infusion was associated with slight and transient effects in plasma chemistry shortly after dosing, such as a brief increase of liver enzyme activity levels, consistent with infusion of a viral protein. Necropsy revealed no significant macroscopic or microscopic abnormalities.

Preliminary data in Hemophilia B mice indicate that treatment with AMT-060 induces normalization of FIX levels as well as clotting time.

- Non-clinical safety & toxicology studies

The following table presents a summary of the AMT-060 non-clinical safety and toxicology studies that are being conducted to support the clinical development program.

Parameter to be assessed	Study performed	Status
[**]	[**]	[**]
[**]	[**]	[**]
[**]	[**]	[**]
[**]	[**]	[**]
[**]	[**]	[**]
[**]	[**]	[**]
[**]	[**]	[**]

Summary of AMT-060 Preclinical Development Program

- AAV5-hFIXco shows similar liver tropism to AAV8-hFIXco and results in significant and long lasting increase in FIX expression.
- Single intravenous administration of AMT-060 into wild type mice and Rhesus macaques results in significant and long lasting hFIX levels with no noticeable adverse events and no macroscopic or microscopic findings.

- GLP safety and toxicology studies are expected to be completed in [\*\*].

Clinical Development Program

The key regulatory and clinical development milestones for AMT-060 include the following,

- EMA Orphan Drug Designation: [\*\*]
- FDA Orphan Drug Designation: [\*\*]
- EMA Scientific Advice: [\*\*]
- EMA Phase I Protocol Advice: [\*\*]
- GLP Safety & Tox Studies: [\*\*]

Expected milestones

- IMPD submission: [\*\*]
- Phase I start: [\*\*]
- Phase II/III start: [\*\*]
- MAA/ NDA submission: [\*\*]
- Phase I trial

The Phase I study will be a multicenter, open label, prospective, interventional, single dose, dose-escalation clinical trial to investigate the safety and tolerability of AAV5-hFIXco (AMT-060) in patients with severe Hemophilia B.

The primary objective is to assess the safety of systemic administration and determine the maximum tolerated doses (MTD). Secondary objectives include:

- To estimate the appropriate dose required to achieve stable expression of hFIX at or above 3% of normal
- To evaluate kinetics (dose-related duration and magnitude) of expression
- To assess the immune response to hFIX transgene product
- To assess the immune response to the AAV5 capsid proteins
- To assess viral shedding in various body fluids (including semen)
- To assess the occurrence of FIX inhibitors
- To evaluate coagulation parameters
- To assess need for FIX concomitant treatment

[\*\*] male adults patients ( $\geq 18$  year old to  $\leq 35$  year old) with genetically confirmed Hemophilia B and phenotypically defined as having severe disease ( $\leq 1\%$  of normal plasma FIX levels) are expected to be enrolled. Initial patient follow-up will last for [\*\*] months as part of the Phase I trial.

- Future Clinical Development

It is envisaged that the Phase II/III will be a confirmatory trial where the study population and the outcomes (efficacy endpoints — clinical and biochemical) will be based on those for the Phase I. **Licensee** will also consider expanding the patient population to moderately severe patients and intend to carry out the study in both Europe and USA.

#### *Summary of AMT-060 Clinical Development Program*

- **The IMPD is planned to be submitted in [\*\*]**
- **Phase I is planned in patients with severe Hemophilia B and is expected to start in [\*\*]**
- **[\*\*]**
- **The Phase II/III program will run in parallel in Europe and USA where MAA and NDA, respectively, are expected in [\*\*]**

The Hemophilia B program has been partnered with Chiesi. The co-development agreement has been shared with NIH.

### **C) Active Research Projects**

#### **1. Hemophilia A**

**Disease Background:** Hemophilia A (HA) is a genetic, X-linked, recessive disorder caused by production of dysfunctional or by production of insufficient amount of factor VIII (FVIII) protein, a key protein involved in the blood coagulation cascade. Hemophilia A patients suffer from spontaneous bleeding in the large joints and soft tissue, and are at risk for intracranial hemorrhage. Recurrent episodes of joint bleeding can lead to crippling arthropathy, particularly in severely affected patients. HA comprises the majority of hemophilia patients (80%), with incidence of  $\sim 1:10,000$  to  $1:50,000$  males affecting 400,000 people worldwide.

Numerous mutations in the FVIII gene have been described giving rise to different disease phenotypes. Similarly to Hemophilia B (HB), individuals with less than 1% active factor are classified as having severe hemophilia, those with 1—5% active factor have moderate hemophilia, and those with mild hemophilia have between 5—40% of normal levels of active clotting factor.

**Clinical need:** HA seems an excellent candidate for gene therapy (GT) as it is a well characterized monogenic disorder. The product of the FVIII gene is a plasma protein which is normally secreted by hepatocytes and endothelial cells but can also be expressed in other cell types, e.g., adipocytes, myocytes or fibroblasts. Furthermore, only modest increase  $>1\%$  can markedly reduce spontaneous bleedings. The effects of gene therapy can be readily monitored by changes in phenotype and by obtaining peripheral blood to measure FVIII antigen levels and clotting factor activity. Currently, treatment for HA consists of infusion of either plasma-derived or rFVIII protein for bleeding episodes. Although, prophylactic infusion of FVIII concentrates is generally effective in alleviating bleeding episodes and subsequent joint disease, the short half-life of FVIII ( $\sim 12$  hours) and the high cost of purified FVIII products make life-long prophylactic treatment demanding for patients and costly.

### **Feasibility**

**Gene:** The gene of factor VIII is located on the long arm of the X chromosome. It spans over 180 kb, and as such is one of the largest genes known. It comprises of 26 exons, which encode a polypeptide chain of 2351 amino acids including a signal peptide of 19 and a mature protein of 2332 amino acids. It is a secreted protein. Its primary structure, deduced from the cloned factor VIII cDNA, includes discrete domain structure: A1-a1-A2-a2-B-a3-A3-C1-C26-8. The B domain is unique in that it exhibits no significant homology with any other known protein and can be deleted with the resulting recombinant protein displaying essentially normal survival in circulation and able to correct the bleeding tendency in HA patients.

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A proof of concept study has been initiated involving a number of FVIII construct and including full FVIII codon optimized gene. The study aims to characterize the viral DNA, formation of episomes upon delivery of the expression cassette to the nucleus, resulting mRNA and FVIII protein. The potency of the vector is currently being investigated in a number of animal models.

It is our aim to develop this product to clinical stage Phase I by the [\*\*]. Duration of clinical development and further timelines have not been defined.

#### **Development overview to IMPD:**

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Completion of vector optimization work will provide the first milestone (Go/No Go) for the project.

**Safety Assessment:** The disease and gene therapy approach are similar (or equivalent) to Hemophilia B where no major safety concerns have been described.

## 2. Cirrhosis

**Disease Background:** Liver cirrhosis is a consequence of chronic liver disease characterized by replacement of liver tissue by fibrosis, scar tissue and regenerative nodules (resulting from regeneration of damaged tissue), leading to loss of liver function. The four leading causes of cirrhosis and primary liver cancer in Europe include harmful alcohol consumption, viral hepatitis B, viral hepatitis C and metabolic syndromes related to overweight and obesity. The European Association for the Study of the Liver in its 2013 report reported that approximately 29 million people in the European Union suffer from a chronic liver condition and that the incidence and prevalence of two conditions, cirrhosis and primary liver cancer, are key to understanding the burden of liver disease. Both conditions represent the end-stage of liver pathology and thus are indicative of the associated mortality.

The hypothesis behind this project is that liver cirrhosis is a state of IGF-I insufficiency and low expression of IGF-I locally in the liver will revert and/ or prevent further exacerbation of cirrhosis. A confidentiality agreement concerning this project was signed between DIGNA/ CIMA and uniQure in October 2012.

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Clinical evidence to support disease linkage includes the following:

- In patients suffering from liver cirrhosis circulating IGF-I levels (or IGF-BP3) correlate with disease severity scores; Child-Pugh and MELD (Kratzsch et al., 2005; Khoshnood et al., 2013).
- A short course (for 4 months) of IGF-I recombinant therapy treatment increased the levels of albumin and tended to improve energy metabolism (surrogates for liver function) & the levels of serum albumin positively correlated with IGF-I/IGF-I BP3 ratio (Conchillo et al., 2005).

**Clinical need:** Transplantation is the only curative option for the disease and contraindications to transplantation include, a) co-morbidities (e.g., TB), b) over 65 years of age, c) coronary artery disease and d) tumours in previous 5 years.

The initial target population for IGF-I gene therapy for liver cirrhosis could/ would be those cirrhotic patients with

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IGF-I insufficiency (i.e., 50% of all cirrhotic patients), possibly patients with Child-Pugh A and/ or B score and with IGF-I levels below normal values. An ODD application for this specific population may be considered. The table below indicates the Child-Pugh scoring scheme for liver disease prognosis.

Points	Class	One year survival	Two year survival
5-6	A	100%	85%
7-9	B	81%	57%
10-15	C	45%	35%

### Feasibility:

**Gene:** The IGF1 gene is located on chromosome 12 and spans 7.3 kb encoding a 70 amino acid residue protein. It contains 6 exons, 4 of which are alternatively spliced depending on tissue type and hormonal environment. The IGF1 coding region is flanked by sequences encoding an amino-terminal peptide of at least 25 residues and a carboxyl-terminal peptide of 35 amino acids which indicates that IGF1 is synthesized as a precursor protein that undergoes proteolytic processing at both ends before being secreted.

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**Animal models:** A rat model is available with CIMA and has been used for proof of concept studies. A number of other small animal models have been described (Liu et al., 2013).

**Biomarkers:** Circulating IGF-I (and other related proteins) can be monitored using commercially available methodology. However the relevance of this to liver (local) levels of IGF-I and whether GT can deliver sufficient amounts of IGF-I that that can be readily detectable in the circulation need to be established.

Liver function and signs of cirrhosis can be monitored following well established standard procedures (e.g., liver enzymes, markers of fibrosis etc.).

The PoC obtained at CIMA will have to be repeated with uniQure's AAV5-IGF1 vector. **Licensee** is at the initial stages of research aiming to initiate a Phase I clinical trial by the [\*\*].

### Development overview to IMPD:

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The GLP safety and toxicology studies will provide the first milestone (Go/No Go) for the project.

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**Safety Assessment:** Safety studies in rat disease models (8 months) and wild type rats (8 weeks) showed no signs of toxicity due to treatment with SV40-IGF-I (Sobrevals et al., 2010).

Potential toxicity concerns include tumor formation and interference with insulin/ glucose metabolism albeit both issues are unlikely as the aim of this approach would be to upregulate levels of IGF-I where they are already below normal rather than to achieve supra-physiological levels. In addition, gene therapy vectors are likely to induce lower level of localized expression without substantial increase in serum IGF-I levels. Regarding potential for tumorigenesis, IGF-I therapy is thought to favor hepatocellular differentiation, i.e., opposes carcinogenesis, and studies have shown that sharp decrease in IGF-I in cirrhotic liver may contribute to hepatocellular carcinoma (HCC). In addition it is believed that it is IGF-II that is the key player in HCC. Furthermore, patients with existing tumor nodules in their liver could/ should be excluded from trials.



[NOTE: Hepatocellular carcinoma occurs at a rate of 1% to 4% per year after cirrhosis is established and cirrhosis underlies HCC in approximately 80%-90% of cases worldwide (Giovanna Fattovich et al., 2004), i.e., the vast majority of cirrhotic patients do not develop HCC or at least they do not live long enough to develop it]

### 3. Hyperoxaluria

**Disease Background:** Primary hyperoxaluria type I (PH1) is a rare, autosomal recessive inherited metabolic disorder characterized by a deficiency of the hepatic enzyme alanine-glyoxylate aminotransferase (AGXT), which produces a marked increase in endogenous oxalate synthesis by the liver. Oxalate is a metabolic end product in humans and excess oxalate provokes hyperoxaluria, causing progressive urolithiasis, nephrocalcinosis and chronic renal failure, ultimately leading to end-stage renal failure (ESRF) and death if untreated.

It is the most common and severe variant among a spectrum of metabolic disorders resulting in hyperoxaluria. The disease has an estimated prevalence ranging from 1 to 3 per 1 million individuals and an estimated incidence of 1-9:100,000 live births per year in Europe. However, higher rates are reported in historically isolated populations, like the Canary Islands. PH1 accounts for <1% of pediatric ESRF in developed countries.

A pre-clinical proof of concept study has already been conducted in collaboration with Eduardo Salido (University Hospital of Canary Islands) using AGXT knockout mice demonstrating that in the GT treated animals oxalurea reduced to normal levels with restoration of liver enzyme levels in the absence of any hepatotoxicity or immune reactions.

**Clinical need:** Currently, most of the therapeutic options are diet-mediated to reduce the amount of glyoxylate intake and maximize the intake of vitamin B6. The most effective treatment for PH1 is pre-emptive liver transplantation, alone or liver combined with kidney transplantation in ESRF. There is therefore a clear need for alternative or new treatments options.

#### Feasibility:

**Gene:** the AGXT gene maps onto chromosome 2q36-q37, has a 10 kb coding sequence and contains 11 exons generating a 392-residue protein.

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**Animal models:** Small animal models already exist and have been used for pre-clinical proof of concept studies.

**Biomarkers:** Measurements of oxalate are part of routine clinical practice for the disease setting and monitoring of kidney changes can also be done using standard techniques.

After a phase of further vector optimization it is our aim to develop this product for a first Phase I clinical study

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by [\*\*]. Further development timelines have not been defined.

#### Development overview to IMPD:

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The GLP safety and toxicology studies will provide the first milestone (Go/No Go) for the project.

**Safety Assessment:** At this stage is not possible to make any inferences in relation to potential safety concerns.

#### Central Nervous System Programs

##### A) Development Programs

##### 1. AMT-110 for Sanfilippo B

#### Disease Background

Sanfilippo syndrome, or Mucopolysaccharidosis type III (MPSIII), is a rare lysosomal storage disorder (LSD) that occurs when enzymes needed to break down the heparan sulfate sugar chain are missing or are defective. Sanfilippo B is one of the four types of MPSIII that results in serious brain degeneration in children, and is generally lethal. The deficient enzyme responsible for the disease is alpha-N-acetylglucosaminidase (NaGlu). The clinical manifestations are mainly neurological, with early symptoms observed during the first five years of age, leading to a progressive deterioration of cognitive abilities. Affected children require specific care after age seven and progressively develop profound mental retardation with reduced somatic manifestations. Death frequently occurs at the median age of 15. No treatment is currently available.

Birth prevalences of 0.28—4.1 per 100, 000 have been reported (Valstar et al., 2008). More recently, He´ron et al. (2010) estimated the mean annual incidence for Sanfilippo B in France at 0.15 per 100,000 births.

#### Overview of AMT-110

The goal of our AMT-110 program is to provide a gene therapy for Sanfilippo B syndrome through the introduction of a functional NaGlu gene into the patients' brain cells.

This project is being pursued together with the Pasteur Institute (Paris) whereby uniQure is responsible for developing the manufacturing process and producing clinical grade material and the Pasteur Institute for conducting the clinical trials.

#### Preclinical Development

- Product Profile

AMT-110 is designed to be delivered via intracranial administration.  
 AMT-110 or rAAV5-hNaGlu, is a recombinant adeno-associated vector of serotype 5, consisting of:

- Inverted terminal regions or ITRs of the adeno-associated serotype 2
- A human  $\alpha$ -N-acetylglucosaminidase, or hNaGlu, gene the therapeutic gene
- The mouse phosphoglycerate kinase-1 promoter (muPGK)
- Pre-clinical Proof of Concept*

Preclinical PoC studies were conducted in mouse and dog disease models at the Pasteur Institute. These studies showed that mice with MSPIIB a single AAV5-NaGlu intracranial injection resulted in reversion of storage lesions throughout the brain and prevented loss of Purkinje cells. Furthermore, it improved animal behavior and corrected pathological featured of the disease including, neuro-inflammation, axonal transport, synaptic vesicle content and the autophagy defect.

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- Non-clinical safety & toxicology studies

The following table presents a summary of the AMT-10 non-clinical safety and toxicology studies that have been conducted to support the clinical development program.

Parameter to be assessed	Study performed	Results
[**]	[**]	[**]
[**]	[**]	[**]
[**]	[**]	[**]

Summary of AMT-110 Preclinical Development Program

- In animal models of Sanfilippo B, treatment with AAV5-hNaGlu ameliorated pathophysiological signs and symptoms of the disease.**

- AMT-110 administered into the striatum of non-immunosuppressed rats and immunosuppressed rats and dogs produced long lasting presence of vector DNA in the brain and caused no mortality and no signs of toxicity.**

Clinical Development Program

The key regulatory and clinical development milestones for AMT-110 include the following,

- 1<sup>st</sup> Scientific Advice with French Regulatory Authorities [\*\*]
- 2<sup>nd</sup> Scientific Advice with French Regulatory Authorities [\*\*]
- IMPD Submission [\*\*]
- IMPD Approval [\*\*]
- Phase I start [\*\*]

Expected Milestones

- Phase II/III start [\*\*]
- Registration [\*\*]

The Phase I/II study is a single center, open label, prospective, interventional, single dose of AAV5-hFIXco (AMT-060) trial in children with Sanfilippo type B syndrome. [\*\*].

The primary objective of the study is to evaluate the clinical, radiological and biological safety of the treatment. The secondary objective is to collect samples and data to define exploratory tests that could become evaluation criteria for further clinical efficacy studies (Brain MRI; neurological tests and biological markers).

The study will be conducted at the Bicêtre Hospital which is part of the University Hospitals of South Paris and is expected to enroll a total of [\*\*] children during an [\*\*] months inclusion period. The duration of follow-up for each patient is [\*\*]. The first patient was dosed in October 2013.

- Future Clinical Development

**Licensee** plans to complete the Phase I and start a Phase II/III trial in multiple sites worldwide. Following initiation of this trial one of the options on how to proceed would be applying for approval for compassionate use to treat on a named patient basis. This can be well justified based on the size of the indication and

- **The IMPD was submitted in [\*\*]**
- **Phase I was started in [\*\*]**

## 2. AMT-090 for Parkinson's Disease

### Disease Background

Parkinson's Disease (PD) is a progressive neurodegenerative disorder that affects motor skills, speech, and other neurological functions. As the condition progresses, every action becomes increasingly difficult, and eventually impossible. The symptoms are caused by degeneration and death of nerve cells in the substantia nigra, a part of the brain that produces dopamine needed to specifically control body movements. Dopamine is a chemical that sends messages in the brain to coordinate and control muscular action and movements. There is currently no cure for Parkinson's Disease, but medications or surgery can provide symptomatic relief, the efficacy of which declines over time and which create significant side effects and co-morbidities, such as depression and dyskinesias. The most widely used treatment is L-dopa in various forms, which is converted to dopamine in the central nervous system. Current symptomatic treatments for Parkinson's Disease represent a multi-billion dollar market.

While medications can temporarily alleviate the symptoms of PD, they do not influence the degenerative process. Progressive loss of nigral dopaminergic (DA) neurons (the pathological hallmark of PD) results in progressive neurologic dysfunction and death. Glial cell line-derived neurotrophic factor (GDNF) was first identified based on its ability to promote the survival of embryonic DA neurons *in vitro*, and research has demonstrated beneficial effects of GDNF in animal models of PD. Recent evidence indicates that gene transfer via direct delivery of viral vectors may represent a superior approach for the treatment of PD with GDNF.

Based on the overwhelming preclinical data of GDNF protective effects on DA neurons, a series of preclinical and clinical studies conducted by third parties have consistently indicated that the infusion of GDNF protein into the brain is effective in Parkinson's Disease. Three clinical trials were performed with direct infusion into the putamen. Two of the studies (Bristol, UK and University of Kentucky) reported favorable clinical response (Gill et al., 2003, Patel et al., 2005, Slevin et al., 2005), and one sponsored by Amgen in the US was abandoned due to apparent lack of efficacy and the appearance of neutralizing antibodies to GDNF in some patients (Lang et al., 2006a). The outcome of these GDNF protein trials still remains controversial (Barker, 2006, Chebrolu et al., 2006, Lang et al., 2006a, Lang et al., 2006b, Penn et al., 2006, Slevin et al., 2006), but the consensus in the scientific community seems to be that the cannula used in the Amgen trial was not optimal, leading to a leakage of the protein into cerebrospinal fluid (CSF). Results from these early clinical trials with GDNF protein underscore the need for a clinical approach in which appropriate levels of GDNF are delivered accurately to the intended sites in the brain where the DA neurons and their terminals reside. Stereotactic parenchymal convection-enhanced delivery of viral vectors carrying the GDNF gene is more likely to achieve precise delivery.

PD is a progressive neurodegenerative disease that advances inexorably over a period of 10 to 30 years to disability and death. Medications, generally those aimed at ameliorating the known striatal dopamine deficiency, can provide substantial benefits for the cardinal symptoms of PD, namely resting tremor, rigidity, bradykinesia and postural instability. Unfortunately, the clinical response wanes over time and a variety of medication-related complications emerge including motor fluctuations, dyskinesias, short duration responses, and psychosis. Disease progression continues since dopamine replacement and other medical therapies have no impact on the underlying neurodegenerative process. Stereotactic deep brain stimulation has emerged as a rational treatment option, but this surgical approach is also symptomatic only and may be associated with serious adverse effects like stroke, hemorrhage, or infection, and hardware-related complications.

### Overview of AMT-090 Program

**Licensee's** AMT-090 program seeks to introduce the gene encoding the GDNF protein to provide a consistent

supply of GDNF to the relevant areas of the brain. Our goal is to inject our AAV2 vector carrying the gene for GDNF into the brain to stop the progression of the disease and possibly measurable clinical and neuroimaging improvement. One of the key elements here is the MRI-guided convection enhanced delivery, which ensures for proper targeting of the vector.

### Preclinical Development

Initial preclinical research was conducted in partnership with the University of Lund, Sweden, which established proof of concept in rodents. Moreover, the University of California San Francisco (UCSF) has conducted many studies consistently demonstrating a therapeutic effect of GDNF in rodents and primates. Key findings include:

- successful gene transfer with AAV2 in the Putamen of rats
- MRI-guided CED delivery of the AAV2 vector to the putamen in non-human primates resulted in GDNF expression in the putamen but also in the substantia nigra
- in a rat lesion model, AAV-GDNF delivery was able to protect neurons from degeneration
- in a primate lesion model, AAV-GDNF delivery was able to protect neurons from degeneration
- no toxicity was observed at any dose levels
- AAV-GDNF was therapeutic in rodent and primate models

- Phase I Clinical Trial

uniQure has entered into an agreement with UCSF and the National Institute of Neurological Diseases and Stroke. Under this agreement, UCSF commenced a Phase I trial of an AAV2 glial cell line-derived neurotrophic factor (GDNF) treatment for Parkinson's Disease in May 2013. This trial is being funded by the National Institutes of Health. **Licensee** has an exclusive right from UCSF to obtain all data related to the program.

The trial includes [\*\*] patients afflicted with advanced Parkinson's Disease (Hoehn and Yahr Stage III or IV off medication) with a Unified PD Rating Scale (UPDRS) (Fahn et al., 1987) total motor score  $\geq 30$  in the defined off state and a serum anti-AAV2 total antibody titer  $< 1000$ .

The study will entail a Phase 1 single-center, open-label, dose escalation, safety and tolerability study of adeno-associated virus, serotype 2 vector (AAV2) containing human GDNF complementary DNA bilaterally delivered by MRI-guided convection-enhanced delivery (CED) to the putamen (450  $\mu$ l per hemisphere) of the. Four escalating dose levels will be evaluated in the following dose cohorts ([\*\*] patients per cohort): [\*\*].

The trial's primary objectives are to assess the safety and tolerability of 4 different dose levels of AAV2-GDNF. The secondary objectives of the trial are to obtain preliminary data regarding the potential for clinical responses of the 4 dose levels tested by assessing the magnitude and variability of any treatment effects (via clinical, laboratory and neuroimaging studies).

- Future Clinical Development

**Licensee** intends to transition this program to our vector and manufacturing platforms and continue the clinical development program. Bridging of the vectors shall include at least testing in Parkinsonian primates and is anticipated to start [\*\*] after the first injections in patients are performed. This will then be followed by a multicenter randomized (delayed) start, blinded, sham-controlled Phase 2 efficacy study of this experimental therapy with our vector. However, prior to finalizing the design of such a trial, **Licensee** propose to conduct a preliminary clinical study that should provide critical information for translating the laboratory research to investigations involving human subjects and critical data for finalizing the ultimate efficacy trial protocol. The preliminary study will also allow us to develop the organizational and logistical processes that will be needed for the anticipated multicenter efficacy trial.

#### *Summary of AMT-090 Clinical Development Program*

- **uniQure has licensed the GDNF gene from Amgen**
- **Pre-clinical PoC studies have been conducted in rodents and non-human primates in partnership with the University of Lund (Sweden) and UCF**
- **A Phase I human trial in Parkinson's disease with AAV2 delivering GDNF has been initiated through a partnership with UCSF**
- **Initiation of first Phase I clinical trial or foreign equivalent — [\*\*]**

Expected milestones

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## **B) Active Research Projects**

### **1. Huntington's Disease**

**Disease background:** Huntington's Disease (HD) is a neurodegenerative genetic disorder that affects motor control and leads to cognitive decline and dementia. It typically becomes noticeable in middle age, but can begin at any age from infancy to old age. HD has a prevalence of around 1 affected individual in 100,000.

The mutated form of the protein huntingtin causes cellular dysfunction and death in a number of CNS sites but is most noticeable in the striatum and cortex. The mutation is caused by CAG repeats in the DNA of patients. The earliest features of HD are involuntary movements and irritability and a loss of executive function. This progresses over time and in the more advanced stages, the patient is demented and bed-bound. The disease is currently incurable with patients dying about 20-25 years after it begins.

**Clinical need:** The clinical need for these patients is high as there is no cure for the disease.

#### **Feasibility**

As the CAG repeats in the Huntingtin gene are the cause of the disease, downregulation of the expression of the CAG repeats is an option. Also rescuing the neurons from degeneration using GDNF is an option. Both options are currently under investigation. Replacing the gene is not an option as this is far too large to fit into an AAV vector.

Several transgenic mice models exist. Severity and time of onset are based on the number of CAG repeats in the model. Mostly used are the R6/1 and R6/2 transgenic models.

**Preclinical work:** Proof of concept using GDNF has been established in one laboratory. **Licensee** is currently trying to establish this with our own vector in the laboratory of Roger Barker.

Proof of concept with siRNA has been established in mice models and **Licensee** is in the process of implementing this into our studies.

#### Development overview to IMPD:

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The proof of concept studies (*in vivo/ in vitro* work) will provide the first milestone (Go/No Go) for the project.

With regards to the siRNA approach to HD, vector generation & optimization will require an additional 9 months prior to any other activity. Then a similar development path to what is shown above will need to be followed.

It is **Licensee's** aim upon a successful PoC to develop this product further to a Phase I clinical investigation which should start [\*\*].

**Collaborators:** **Licensee** is working together with Roger Barker (Cambridge University) on the use of GDNF to rescue neurons in Huntington models, based on a EUREKA grant. **Licensee** is also working together with Nicole Deglon (Lausanne University), Anna Skorupska (Lublin University) and Sebastian Kuegler (Gottingen University) in a Eurostars grant setting. Competition comes from siRNA companies.

**Safety concerns:** Potential safety concerns could be the complete downregulation of the Huntingtin gene, even though not fully supported by the Eurostars team. The use of GDNF could lead to side effects, such as weight loss.

**IP:** For GDNF, Licensee has a license from Amgen. For the siRNA work **Licensee** has a non-exclusive license from

Benitec.

## 2. Multiple System Atrophy

**Disease Background:** Multiple System Atrophy (MSA) is a sporadic neurodegenerative disease that is characterized by the presence of glial inclusion bodies, which stain positive for a synuclein. The clinical picture is that of parkinsonism, autonomic failure, cerebellar ataxia and pyramidal signs in differing combinations. Approximately 80% of patients present with predominantly parkinsonian features (MSA-P) manifesting in rapidly deteriorating akinesia, rigidity, postural instability and high pitched dysarthria. Most such patients do not exhibit the classic resting tremor of Parkinson's disease and virtually all develop frank dysautonomia in the course of the illness. The cause of the disease is not known.

**Clinical need:** Although a minority of patients may achieve modest benefit from dopaminergic therapy, there is no satisfactory treatment for the parkinsonian disabilities of MSA-P. Additionally, deep brain stimulation of the subthalamic nucleus has been of little or no value. Within 5 years of disease onset patients die so the clinical need is high for these patients.

#### Feasibility:

MSA is not a single monogenic disease, but may be treated with a single neuroprotective protein. In this case, this could be GDNF. Some transgenic animal models exist, all overexpressing the alpha-synuclein protein. The rationale to use GDNF (besides its general neuroprotective effect on neurons) is that both in patients and the transgenic mouse model, GDNF expression is downregulated. Introduction of an elevated level of GDNF may serve as the treatment. Read out parameters for the disease progression are all related to those of Parkinson's Disease. PoC has not yet been established, but is under investigation in the mouse model.

#### Development overview to IMPD:

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The proof of concept studies (*in vivo/ in vitro* work) will provide the first milestone (Go/No Go) for the project.

It is our aim upon a successful PoC to develop this product further to a Phase I clinical investigation which should start [\*\*].

**Collaborators:** **Licensee** is working together with Erwan Bezard (University of Bordeaux) and Olivier Rascol (University of Toulouse) who are together running the French reference center for MSA.

**Safety Assessment:** The use of GDNF could lead to side effects, such as weight loss. The exact mechanism through which the treatments would have its effect is not clear yet.

## 3. Hearing loss

**Disease background:** Hearing loss is a serious clinical problem. Underlying mechanisms for the loss of neurons in the cochlea can vary from ischemia, mechanical stress to toxic insults. The actual numbers of patients is not easy to define, but it could be rather large. When age-related hearing loss is also taken into account, this is no longer an orphan indication.

**Clinical need:** Patients with hearing loss could be helped with cochlear implants. However, progressive neurodegeneration is not stopped by that. There is high clinical need as there is no cure for the disease.

#### Feasibility:

Neuron function and survival is dependent on a delicate balance of neurotrophins. Following trauma or toxic insult to neurons, they may slowly die. To reverse this state of degeneration, it could be beneficial to supply the neurons with a neurotrophin such as GDNF. This neurotrophin has been shown to be able to

rescue neurons from degeneration in several models, including those of the substantia nigra and for instance motoneurons in the spinal cord after trauma.

Animal models are available and include for instance use of Kanamycin in cats, mice or guinea pigs. Also chemotherapeutic agents from the class of statins are used.

**Preclinical work:** Proof of concept using recombinant brain-derived neurotrophic factor (BDNF) and/or GDNF has been established. **Licensee** is currently trying to establish this with our own vector in the laboratory of Patricia Leake.

Cochlea of mice can be transduced to express a recombinant transgene.

**Development overview to IMPD:**

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The proof of concept studies (*in vivo/ in vitro* work) will provide the first milestone (Go/No Go) for the project.

This new project has just been initiated upon a successful PoC it is our aim to develop this product further to a Phase I clinical trial, which should start by the [\*\*].

**Collaborators:** **Licensee** is working together with Patricia Leake (University College of San Francisco) on the use of GDNF to rescue neurons in mouse and cat models. She is the investigator who developed the cochlear implant. This could also be included in the experimental plan.

**Safety concerns:** The use of GDNF could lead to side effects. Weight loss is not expected, but as the GDNF also has a neurotrophic effect, nerve fibers could sprout in an aberrant way possibly leading to incorrect connections.

**IP:** For GDNF, **Licensee** has a license from Amgen; the program as a whole is under investigation.

**Exploratory Research Projects**

The projects listed under this category in Table 1 above are not in active research yet, but are likely targets for our platform technology and are being assessed on feasibility before starting active bench work.

Confidential Materials omitted and filed separately with the Securities and Exchange Commission. Double asterisks denote omissions.

**PUBLIC HEALTH SERVICE**  
**PATENT LICENSE AGREEMENT - *EXCLUSIVE and NON-EXCLUSIVE***

COVER PAGE

For PHS internal use only:

License Number:  
**L-116-2011/0**

License Application Number: A-063-2009

Serial Number(s) of Licensed Patent(s) or Patent Application(s):

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Licensee: Amsterdam Molecular Therapeutics (AMT) B.V.

Cooperative Research and Development Agreement (CRADA) Number (if a subject invention): N/A

Additional Remarks: This Patent License Agreement will replace **PHS** license L-119-2007/0 and any amendments thereto.

Public Benefit(s): Commercialization of this technology will benefit the public health by providing AAV5 based gene therapies to treat diseases originated from the brain and liver.

This Patent License Agreement, hereinafter referred to as the “**Agreement**”, consists of this Cover Page, an attached **Agreement**, a Signature Page, Appendix A (List of Patent(s) or Patent Application(s)), Appendix B (Fields of Use and Territory), Appendix C (Royalties), Appendix D (Benchmarks and Performance), Appendix E (Commercial Development Plan), Appendix F (Example Royalty Report), and Appendix G (Royalty Payment Options). The Parties to this **Agreement** are:

- 1) The National Institutes of Health (“**NIH**”) or the Food and Drug Administration (“**FDA**”), hereinafter singly or collectively referred to as “**PHS**”, agencies of the United States Public Health Service within the Department of Health and Human Services (“**HHS**”); and
- 2) The person, corporation, or institution identified above or on the Signature Page, having offices at the address indicated on the Signature Page, hereinafter referred to as “**Licensee**”.

A-063-2009

**CONFIDENTIAL**

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**PHS PATENT LICENSE AGREEMENT - *EXCLUSIVE and NON-EXCLUSIVE***

**PHS** and **Licensee** agree as follows:

1. **BACKGROUND**

- 1.1 In the course of conducting biomedical and behavioral research, **PHS** investigators made inventions that may have commercial applicability.
- 1.2 By assignment of rights from **PHS** employees and other inventors, **HHS**, on behalf of the **Government**, owns intellectual property rights claimed in any United States or foreign patent applications or patents corresponding to the assigned inventions. **HHS** also owns any tangible embodiments of these inventions actually reduced to practice by **PHS**.
- 1.3 The Secretary of **HHS** has delegated to **PHS** the authority to enter into this **Agreement** for the licensing of rights to these inventions.
- 1.4 **PHS** desires to transfer these inventions to the private sector through commercialization licenses to facilitate the commercial development of products and processes for public use and benefit.
- 1.5 **Licensee** desires to acquire commercialization rights to certain of these inventions in order to develop processes, methods, or marketable products for public use and benefit.

2. **DEFINITIONS**

- 2.1 “**Affiliate(s)**” means a corporation or other business entity, which directly or indirectly is controlled by or controls, or is under common control with **Licensee**. For this purpose, the term “control” shall mean ownership of more than fifty percent (50%) of the voting stock or other ownership interest of the corporation or other business entity, or the power to elect or appoint more than fifty percent (50%) of the members of the governing body of the corporation or other business entity.

- 2.2 “**Benchmarks**” mean the performance milestones that are set forth in Appendix D.
- 2.3 “**Commercial Development Plan**” means the written commercialization plan attached as Appendix E.
- 2.4 “**Exempt Collaborator**” means a not-for-profit organization or academic institution that has entered into a formal collaboration and / or supply agreement with **Licensee** to conduct pre-clinical development and solely sponsor clinical trials of **Licensed Product**, excluding **Supplied Materials**, to treat an **Ultra-Orphan Indication**; in which **Licensee** may acquire clinical development and data for regulatory approval and sale of a **Licensed Product**.
- 2.5 “**First Commercial Sale**” means the initial transfer by or on behalf of **Licensee** or its sublicensees of **Licensed Products** or the initial practice of a **Licensed Process** by or on behalf of **Licensee** or its sublicensees in exchange for cash or some equivalent to which value can be assigned for the purpose of determining **Net Sales**.
- 2.6 “**Government**” means the Government of the United States of America.
- 2.7 “**Licensed Fields of Use**” means the fields of use a) and b) as identified in Appendix B.
- 2.8 “**Licensed Patent Rights**” shall mean:

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- (a) Patent applications (including provisional patent applications and PCT patent applications) or patents listed in Appendix A, all divisions and continuations of these applications, all patents issuing from these applications, divisions, and continuations, and any reissues, reexaminations, and extensions of these patents;
- (b) to the extent that the following contain one or more claims directed to the invention or inventions disclosed in 2.8(a):
- (i) continuations-in-part of 2.8(a);
- (ii) all divisions and continuations of these continuations-in-part;
- (iii) all patents issuing from these continuations-in-part, divisions, and continuations;
- (iv) priority patent application(s) of 2.8(a); and
- (v) any reissues, reexaminations, and extensions of these patents;
- (c) to the extent that the following contain one or more claims directed to the invention or inventions disclosed in 2.8(a): all counterpart foreign and U.S. patent applications and patents to 2.8(a) and 2.8(b), including those listed in Appendix A; and
- (d) **Licensed Patent Rights** shall not include 2.8(b) or 2.8(c) to the extent that they contain one or more claims directed to new matter which is not the subject matter disclosed in 2.8(a).
- 2.9 “**Licensed Processes**” means processes which, in the course of being practiced, would be within the scope of one or more claims of the **Licensed Patent Rights** that have not been held unpatentable, invalid or unenforceable by an unappealed or unappealable judgment of a court of competent jurisdiction.
- 2.10 “**Licensed Products**” means tangible materials which, in the course of manufacture, use, sale, or importation, would be within the scope of one or more claims of the **Licensed Patent Rights** that have not been held unpatentable, invalid or unenforceable by an unappealed or unappealable judgment of a court of competent jurisdiction.
- 2.11 “**Licensed Territory**” means the geographical area identified in Appendix B.
- 2.12 “**Marketing Approval**” means any and all approvals (including price and reimbursement approvals, if required), licenses, registrations, or authorizations of regulatory authorities in any country that are necessary for the manufacture, use, storage, import, transport and/or sale of a **Licensed Product** in the Licensed Fields of Use in such country.
- 2.13 “**Net Sales**” means the total gross receipts for sales of **Licensed Products** or practice of **Licensed Processes** by or on behalf of **Licensee** or its sublicensees, and from leasing, renting, or otherwise making **Licensed Products** available to others without sale or other dispositions, whether invoiced or not, less returns and allowances, packing costs, insurance costs, freight out, taxes or excise duties imposed on the transaction (if separately invoiced), and wholesaler and cash discounts in amounts customary in the trade to the extent actually granted. No deductions shall be made for commissions paid to individuals, whether they are with independent sales agencies or regularly employed by **Licensee**, or sublicensees, and on its payroll, or for the cost of collections.

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- 2.14 “**Orphan Indication**” means a disease that affects less than two hundred thousand (200,000) people in the United States as defined by the Food and Drug Administration or five (5) in ten thousand (10,000) people in the European Union as defined by the European Medicines Agency.
- 2.15 “**Practical Application**” means to manufacture in the case of a composition or product, to practice in the case of a process or method, or to operate in the case of a machine or system; and in each case, under these conditions as to establish that the invention is being utilized and that its benefits are to the extent permitted by law or **Government** regulations available to the public on reasonable terms.



- 2.16 “**Research License**” means a nontransferable, nonexclusive license to make and to use **Licensed Products** or **Licensed Processes** as defined by the **Licensed Patent Rights** for purposes of research and not for purposes of commercial manufacture or distribution or in lieu of purchase.
- 2.17 “**Supplied Materials**” means [\*\*]. Further, these **Supplied Materials** were supplied by **PHS** to **Licensee** under a Material Transfer Agreement.
- 2.18 “**Third Party Applicant**” shall mean any non-**Licensee** applicant from whom **PHS** receives a license application for **Licensed Patent Rights** in an indication for which proposed commercial development is not addressed in **Licensee**’s then current **Commercial Development Plan** outlined in Appendix E of this **Agreement**.
- 2.19 “**Ultra-Orphan Indication**” means a disease that affects less than one (1) in Fifty Thousand (50,000) people in the United States or the European Union.

### 3. GRANT OF RIGHTS

- 3.1 **PHS** hereby grants and **Licensee** accepts, subject to the terms and conditions of this **Agreement**, an exclusive license and non-exclusive license, as specified in Appendix B, under the **Licensed Patent Rights** in the **Licensed Territory** to make and have made, to use and have used, to sell and have sold, to offer to sell, and to import any **Licensed Products** in the **Licensed Fields of Use** and to practice and have practiced any **Licensed Processes** in the **Licensed Fields of Use**.
- 3.2 This **Agreement** confers no license or rights by implication, estoppel, or otherwise under any patent applications or patents of **PHS** other than the **Licensed Patent Rights** regardless of whether these patents are dominant or subordinate to the **Licensed Patent Rights**.

### 4. SUBLICENSING

- 4.1 Upon written approval, which shall include prior review of any sublicense agreement by **PHS** and which shall not be unreasonably withheld, **Licensee** may enter into sublicensing agreements under the **Licensed Patent Rights**.
- 4.2 **Licensee** agrees that any sublicenses granted by it shall provide that the obligations to **PHS** of Paragraphs 5.1-5.4, 8.1, 10.1, 10.2, 12.5, and 13.8-13.10 of this **Agreement** shall be binding upon the sublicensee as if it were a party to this **Agreement**. **Licensee** further agrees to attach copies of these Paragraphs to all sublicense agreements.
- 4.3 Any sublicenses granted by **Licensee** shall provide for the termination of the sublicense, or the conversion to a license directly between the sublicensees and **PHS**, at the option of the sublicensee, upon termination of this **Agreement** under Article 13. This conversion is subject to **PHS** approval (not to be unreasonably withheld) and contingent upon acceptance by the sublicensee of the remaining provisions of this **Agreement**.

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- 4.4 **Licensee** agrees to forward to **PHS** a complete copy of each fully executed sublicense agreement postmarked within [\*\*] days of the execution of the agreement. To the extent permitted by law, **PHS** agrees to maintain each sublicense agreement in confidence.

### 5. STATUTORY AND PHS REQUIREMENTS AND RESERVED GOVERNMENT RIGHTS

- 5.1 (a) **PHS** reserves on behalf of the **Government** an irrevocable, nonexclusive, nontransferable, royalty-free license for the practice of all inventions licensed under the **Licensed Patent Rights** throughout the world by or on behalf of the **Government** and on behalf of any foreign government or international organization pursuant to any existing or future treaty or agreement to which the **Government** is a signatory. Prior to the **First Commercial Sale**, **Licensee** agrees to provide **PHS** with reasonable quantities of **Licensed Products** or materials made through the **Licensed Processes** for **PHS** research use; and
- (b) In the event that the **Licensed Patent Rights** are Subject Inventions made under a Cooperative Research and Development Agreement (“**CRADA**”), **Licensee** grants to the **Government**, pursuant to 15 U.S.C. §3710a(b)(1)(A), a nonexclusive, nontransferable, irrevocable, paid-up license to practice **Licensed Patent Rights** or have **Licensed Patent Rights** practiced throughout the world by or on behalf of the **Government**. In the exercise of this license, the **Government** shall not publicly disclose trade secrets or commercial or financial information that is privileged or confidential within the meaning of 5 U.S.C. §552(b)(4) or which would be considered as such if it had been obtained from a non-Federal party. Prior to the **First Commercial Sale**, **Licensee** agrees to provide **PHS** reasonable quantities of **Licensed Products** or materials made through the **Licensed Processes** for **PHS** research use.
- 5.2 **Licensee** agrees that products used or sold in the United States embodying **Licensed Products** or produced through use of **Licensed Processes** shall be manufactured substantially in the United States, unless a written waiver is obtained in advance from **PHS**.
- 5.3 **Licensee** acknowledges that **PHS** may enter into future **CRADAs** under the Federal Technology Transfer Act of 1986 that relate to the subject matter of this **Agreement**. **Licensee** agrees not to unreasonably deny requests for a **Research License** from future collaborators with **PHS** when acquiring these rights is necessary in order to make a **CRADA** project feasible. **Licensee** may request an opportunity to join as a party to the proposed **CRADA**.
- 5.4 (a) In addition to the reserved license of Paragraph 5.1, **PHS** reserves the right to grant **Research Licenses** directly or to require **Licensee** to grant **Research Licenses** on reasonable terms. The purpose of these **Research Licenses** is to encourage basic research, whether conducted at an academic or corporate facility. In order to safeguard the **Licensed Patent Rights**, however, **PHS** shall consult with **Licensee** before granting to commercial entities a **Research License** or providing to them research samples of materials made through the **Licensed Processes**; and
- (b) In exceptional circumstances, and in the event that **Licensed Patent Rights** are Subject Inventions made under a **CRADA**, the **Government**, pursuant to 15 U.S.C. §3710a(b)(1)(B), retains the right to require the **Licensee** to grant to a responsible applicant a nonexclusive, partially exclusive, or exclusive sublicense to use the **Licensed Patent Rights** in the **Licensed Field of Use** on terms that are reasonable under the circumstances, or if **Licensee** fails to grant this license, the **Government** retains the right to grant the license itself. The exercise of these rights by the **Government** shall only be in exceptional circumstances and only if the **Government** determines:

- (i) the action is necessary to meet health or safety needs that are not reasonably satisfied by **Licensee**;
  - (ii) the action is necessary to meet requirements for public use specified by Federal regulations, and these requirements are not reasonably satisfied by the **Licensee**; or
  - (iii) the **Licensee** has failed to comply with an agreement containing provisions described in 15 U.S.C. §3710a(c)(4)(B); and
- (c) The determination made by the **Government** under this Paragraph 5.4 is subject to administrative appeal and judicial review under 35 U.S.C. §203(b).

6. ROYALTIES AND REIMBURSEMENT

- 6.1 **Licensee** agrees to pay **PHS** a noncreditable, nonrefundable license issue royalty as set forth in Appendix C.
- 6.2 **Licensee** agrees to pay **PHS** a nonrefundable minimum annual royalty as set forth in Appendix C.
- 6.3 Unless otherwise exempted in Paragraphs 6.13-6.19, **Licensee** agrees to pay **PHS** earned royalties as set forth in Appendix C.
- 6.4 Unless otherwise exempted in Paragraphs 6.13-6.19, **Licensee** agrees to pay **PHS** benchmark royalties as set forth in Appendix C.
- 6.5 **Licensee** agrees to pay **PHS** sublicensing royalties as set forth in Appendix C.
- 6.6 A patent or patent application licensed under this **Agreement** shall cease to fall within the **Licensed Patent Rights** for the purpose of computing earned royalty payments in any given country on the earliest of the dates that:
- (a) the application has been abandoned and not continued;
  - (b) the patent expires or irrevocably lapses, or
  - (c) the patent has been held to be invalid or unenforceable by an unappealed or unappealable decision of a court of competent jurisdiction or administrative agency.
- 6.7 No multiple royalties shall be payable because any **Licensed Products** or **Licensed Processes** are covered by more than one of the **Licensed Patent Rights**. In the event that this **Agreement** and **PHS** license L-107-2007/0 as amended from time to time apply to the same product sold by the **Licensee** or its sublicensees then the **Licensee** shall only pay earned royalties and benchmark royalties under this **Agreement**.
- 6.8 On sales of **Licensed Products** by **Licensee** to sublicensees or on sales made in other than an arms-length transaction, the value of the **Net Sales** attributed under this Article 6 to this transaction shall be that which would have been received in an arms-length transaction, based on sales of like quantity and quality products on or about the time of this transaction.
- 6.9 With regard to unreimbursed expenses associated with the preparation, filing, prosecution, and maintenance of all patent applications and patents included within the **Licensed Patent Rights** and

paid by **PHS** prior to the effective date of this **Agreement**. **Licensee** shall pay **PHS**, as an additional royalty, on or before March 1, 2012, and upon **PHS**' submission of a statement and request for payment to **Licensee**, an amount equivalent to these unreimbursed expenses previously paid by **PHS**, the total amount should not exceed **[\*\*]** U.S. dollars (\$**[\*\*]**). If this **Agreement** is terminated by **Licensee** on or before March 1, 2012, **Licensee** agrees to pay the amount in full within **[\*\*]** days before termination.

- 6.10 With regard to unreimbursed expenses associated with the preparation, filing, prosecution, and maintenance of all patent applications and patents included within the **Licensed Patent Rights** and paid by **PHS** on or after the effective date of this **Agreement**. **PHS**, at its sole option, may require **Licensee**:
- (a) to pay **PHS** on an annual basis, within **[\*\*]** days of **PHS**' submission of a statement and request for payment, a royalty amount equivalent to these unreimbursed expenses paid during the previous calendar year;
  - (b) to pay these unreimbursed expenses directly to the law firm employed by **PHS** to handle these functions. However, in this event, **PHS** and not **Licensee** shall be the client of the law firm; or
  - (c) in limited circumstances, **Licensee** may be given the right to assume responsibility for the preparation, filing, prosecution, or maintenance of any patent application or patent included with the **Licensed Patent Rights**. In that event, **Licensee** shall directly pay the attorneys or agents engaged to prepare, file, prosecute, or maintain these patent applications or patents and shall provide **PHS** with copies of each invoice associated with these services as well as documentation that these invoices have been paid.
- 6.11 **PHS** agrees, upon written request, to provide **Licensee** with summaries of patent prosecution invoices for which **PHS** has requested payment from the **Licensee** under Paragraphs 6.9 and 6.10. **Licensee** agrees that all information provided by **PHS** related to patent prosecution costs shall be treated as confidential commercial information and shall not be released to a third party except as required by law or a court of competent jurisdiction.

6.12 **Licensee** may elect to surrender its rights in any country of the **Licensed Territory** under any of the **Licensed Patent Rights** upon **[\*\*]** days written notice to **PHS** and owe no payment obligation under Paragraph 6.10 for patent-related expenses paid in that country after **[\*\*]** days of the effective date of the written notice.

6.13 Exemption for **Ultra-Orphan Indication** Research

- (a) **Licensee** shall be permitted, upon **PHS** consent, (not to be unreasonably withheld), to manufacture and supply **Licensed Product**, excluding **Supplied Materials**, to an **Exempt Collaborator** for use solely in pre-clinical and clinical development to treat an **Ultra-Orphan Indication**. Prior to commencement of manufacturing of **Licensed Product** for an **Exempt Collaborator**, **Licensee** shall request permission in writing and must obtain written consent from **PHS**. Additional documentation to establish an **Exempt Collaborator** may be required by **PHS**.
- (b) For avoidance of doubt, **Licensee** shall retain **Supplied Materials** and shall not release **Supplied Materials** alone to an **Exempt Collaborator**.
- (c) Upon receipt of written consent from **PHS** for manufacturing of a **Licensed Product** for an **Exempt Collaborator**. **Licensee** shall not be obligated to pay **Benchmark** royalties

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which would have been payable under **Appendix C**, Section IV for **Benchmarks** triggered by clinical trials solely sponsored by the **Exempt Collaborator** until such time as **Licensee** exercises its option to acquire the clinical development from the **Exempt Collaborator**.

- (d) Upon acquisition of the clinical development from an **Exempt Collaborator**. **Licensee** shall pay **PHS** royalties which become payable from that point onwards in accordance with **Appendix C**, Section IV. **Licensee** must inform **PHS** in writing within **[\*\*]** days of **Licensee's** decision to acquire or not acquire clinical development from the **Exempt Collaborator**.
- (e) For avoidance of doubt, **PHS** shall consider **Licensee's** sponsorship or co-sponsorship of a clinical trial or regulatory submission for a **Licensed Product** to treat an **Ultra-Orphan Indication** as an acquisition of clinical development from an **Exempt Collaborator**.
- (f) Earned royalty payments on **Net Sales** specified in **Appendix C**, Section III shall not be applicable to **Licensed Product** manufactured for research and clinical trials conducted by an **Exempt Collaborator** approved by **PHS** per Paragraph 6.13.
- (g) In lieu of earned royalty payments, **Licensee** shall pay **PHS** a royalty payment of **[\*\*]** U.S. dollars (\$**[\*\*]**) for each collaboration approved by **PHS** with an **Exempt Collaborator**. Such royalty shall be due within **[\*\*]** days of the date of **PHS** written consent per Paragraph 6.13. In the event that several licenses granted by **PHS** to the **Licensee** apply to the same product, only a single payment of \$**[\*\*]** will be payable per collaboration.

7. PATENT FILING, PROSECUTION, AND MAINTENANCE

- 7.1 Except as otherwise provided in this Article 7, **PHS** agrees to take responsibility for, but to consult with, the **Licensee** in the preparation, filing, prosecution, and maintenance of any and all patent applications or patents included in the **Licensed Patent Rights** and shall furnish copies of relevant patent-related documents to **Licensee**.
- 7.2 Upon **PHS'** written request, **Licensee** shall assume the responsibility for the preparation, filing, prosecution, and maintenance of any and all patent applications or patents included in the **Licensed Patent Rights** and shall, on an ongoing basis, promptly furnish copies of all patent-related documents to **PHS**. In this event, **Licensee** shall, subject to the prior approval of **PHS**, select registered patent attorneys or patent agents to provide these services on behalf of **Licensee** and **PHS**. **PHS** shall provide appropriate powers of attorney and other documents necessary to undertake this action to the patent attorneys or patent agents providing these services. **Licensee** and its attorneys or agents shall consult with **PHS** in all material aspects of the preparation, filing, prosecution and maintenance of patent applications and patents included within the **Licensed Patent Rights** and shall provide **PHS** sufficient opportunity to comment on any document that **Licensee** intends to file or to cause to be filed with the relevant intellectual property or patent office.
- 7.3 At any time, **PHS** may provide **Licensee** with written notice that **PHS** wishes to assume control of the preparation, filing, prosecution, and maintenance of any and all patent applications or patents included in the **Licensed Patent Rights** such that the terms of Paragraph 7.1 shall then apply. If **PHS** elects to reassume these responsibilities, **Licensee** agrees to cooperate fully with **PHS**, its attorneys, and agents in the preparation, filing, prosecution, and maintenance of any and all patent applications or patents included in the **Licensed Patent Rights** and to provide **PHS** with complete copies of any and all documents or other materials that **PHS** deems necessary to undertake such responsibilities. **Licensee** shall be responsible for all costs associated with transferring patent prosecution responsibilities to an attorney or agent of **PHS'** choice.

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- 7.4 Each party shall promptly inform the other as to all matters that come to its attention that may materially affect the preparation, filing, prosecution, or maintenance of the **Licensed Patent Rights** and permit each other to provide comments and suggestions with respect to the preparation, filing, prosecution, and maintenance of **Licensed Patent Rights**, which comments and suggestions shall be considered by the other party.

8. RECORD KEEPING

- 8.1 **Licensee** agrees to keep accurate and correct records of **Licensed Products** made, used, sold, or imported and **Licensed Processes** practiced under this **Agreement** appropriate to determine the amount of royalties due **PHS**. These records shall be retained for at least **[\*\*]** years following a given reporting period and shall be available during normal business hours for inspection, at the expense of **PHS**, by an accountant selected by **PHS** for the sole purpose of verifying reports and royalty payments hereunder. The accountant shall only disclose to **PHS**

information relating to the accuracy of reports and royalty payments made under this **Agreement**. Such inspections may be made no more than [\*\*], with reasonable efforts to minimize disruption of **Licensee's** normal business activities. Such records for any particular calendar quarter shall be subject to no more than [\*\*]. If an inspection shows an underreporting or underpayment in excess of [\*\*] percent ([\*\*]%) for any [\*\*] period, then **Licensee** shall reimburse **PHS** for the cost of the inspection at the time **Licensee** pays the unreported royalties, including any additional royalties as required by Paragraph 9.8. All royalty payments required under this Paragraph shall be due within [\*\*] days of the date **PHS** provides **Licensee** notice of the payment due.

## 9. REPORTS ON PROGRESS, BENCHMARKS, SALES, AND PAYMENTS

- 9.1 Prior to signing this **Agreement**, **Licensee** has provided **PHS** with the **Commercial Development Plan** in Appendix E, under which **Licensee** intends to bring the subject matter of the **Licensed Patent Rights** to the point of **Practical Application**. This **Commercial Development Plan** is hereby incorporated by reference into this **Agreement**. Based on this plan, performance **Benchmarks** are determined as specified in Appendix D.
- 9.2 **Licensee** shall provide written annual reports on its product development progress or efforts to commercialize under the **Commercial Development Plan** for each of the **Licensed Fields of Use** within [\*\*] days after December 31 of each calendar year. These progress reports shall include, but not be limited to: progress on research and development, status of applications for regulatory approvals, manufacturing, sublicensing, marketing, importing, and sales during the preceding calendar year, as well as, plans for the present calendar year. **PHS** also encourages these reports to include information on any of **Licensee's** public service activities that relate to the **Licensed Patent Rights**. If reported progress differs from that projected in the **Commercial Development Plan** and **Benchmarks**, **Licensee** shall explain the reasons for these differences. In the annual report, **Licensee** may propose amendments to the **Commercial Development Plan**, acceptance of which by **PHS** may not be denied unreasonably. **Licensee** agrees to provide any additional information reasonably required by **PHS** to evaluate **Licensee's** performance under this **Agreement**. **Licensee** may amend the **Benchmarks** at any time upon written approval by **PHS**. **PHS** shall not unreasonably withhold approval of any request of **Licensee** to extend the time periods of this schedule if the request is supported by a reasonable showing by **Licensee** of diligence in its performance under the **Commercial Development Plan** and toward bringing the **Licensed Products** to the point of **Practical Application** as defined in 37 C.F.R. §404.3(d). **Licensee** shall amend the **Commercial Development Plan** and **Benchmarks** at the request of **PHS** to address any **Licensed Fields of Use** not specifically addressed in the plan originally submitted.
- 9.3 **Licensee** shall report to **PHS** the dates for achieving **Benchmarks** specified in Appendix D and the **First Commercial Sale** in each country in the **Licensed Territory** within [\*\*] days of such occurrences.
- 9.4 **Licensee** shall submit to **PHS**, within [\*\*] days after each calendar half-year ending June 30 and December 31, a royalty report, as described in the example in Appendix F, setting forth for the preceding half-year period the amount of the **Licensed Products** sold or **Licensed Processes** practiced by or on behalf of **Licensee** in each country within the **Licensed Territory**, the **Net Sales**, and the amount of royalty accordingly due. With each royalty report, **Licensee** shall submit payment of earned royalties due. If no earned royalties are due to **PHS** for any reporting period, the written report shall so state. The royalty report shall be certified as correct by an authorized officer of **Licensee** and shall include a detailed listing of all deductions made under Paragraph 2.13 to determine **Net Sales** made under Article 6 to determine royalties due.
- 9.5 **Licensee** agrees to forward semi-annually to **PHS** a copy of these reports received by **Licensee** from its sublicensees during the preceding half-year period as shall be pertinent to a royalty accounting to **PHS** by **Licensee** for activities under the sublicense.
- 9.6 Royalties due under Article 6 shall be paid in U.S. dollars and payment options are listed in Appendix G. For conversion of foreign currency to U.S. dollars, the conversion rate shall be the New York foreign exchange rate quoted in *The Wall Street Journal* on the day that the payment is due. Any loss of exchange, value, taxes, or other expenses incurred in the transfer or conversion to U.S. dollars shall be paid entirely by **Licensee**. The royalty report required by Paragraph 9.4 shall be mailed to **PHS** at its address for **Agreement** Notices indicated on the Signature Page.
- 9.7 **Licensee** shall be solely responsible for determining if any tax on royalty income is owed outside the United States and shall pay the tax and be responsible for all filings with appropriate agencies of foreign governments.
- 9.8 Additional royalties may be assessed by **PHS** on any payment that is more than [\*\*] days overdue at the rate of [\*\*] percent ([\*\*]%) per month. This [\*\*] percent ([\*\*]%) per month rate may be applied retroactively from the original due date until the date of receipt by **PHS** of the overdue payment and additional royalties. The payment of any additional royalties shall not prevent **PHS** from exercising any other rights it may have as a consequence of the lateness of any payment.
- 9.9 All plans and reports required by this Article 9 and marked "confidential" by **Licensee** shall, to the extent permitted by law, be treated by **PHS** as commercial and financial information obtained from a person and as privileged and confidential, and any proposed disclosure of these records by the **PHS** under the Freedom of Information Act (FOIA), 5 U.S.C. §552 shall be subject to the predisclosure notification requirements of 45 C.F.R. §5.65(d).
- 9.10 In the event **PHS** receives a license application from a **Third Party Applicant** for commercial development of one or more **Licensed Products** or **Licensed Processes** in the exclusive **Licensed Fields of Use**, as they pertain to **Licensed Patent Rights** for which the proposed commercial development is not specifically addressed in **Licensee's** then-current **Commercial Development Plan** ("**Third Party Applications**"), **PHS** shall notify **Licensee**, in writing, of the existence of the **Third Party Applicant's** license application. Upon receipt of the written notice, **Licensee** shall respond in writing by either: (a) amending its **Commercial Development Plan** within [\*\*] days in a manner acceptable to **PHS** to include a clinical research and development program for the proposed commercial development of the **Third Party Applications** including revised **Benchmarks** to be incorporated into Appendix E, and acceptance of the amendment to the **Commercial Development Plan** by **PHS** shall take into account if **Licensee** has already carried out work in respect of such **Third Party Applications** prior to notification by **PHS**; or (b) amending its **Commercial**

**Development Plan** within [\*\*] days (or such longer period agreed by **Licensee** and such **Third Party Applicant**) in a manner acceptable to **PHS** to include a joint pre-clinical research and development program with the **Third Party Applicant** for the proposed commercial development of the **Third Party Applications**; or (c) granting an exclusive or non-exclusive sublicense under commercially reasonable terms to the **Third Party Applicant** under **Licensed Patent Rights** in respect of the **Third Party Applications** within [\*\*] days (or such longer period agreed by **Licensee** and such **Third Party Applicant**); or both (b) and (c). If **Licensee** does not respond to the written notice as described in this Paragraph 9.10, and after [\*\*] days of final notice being sent to **Licensee**, **PHS** may remove the **Licensed Products** or **Licensed Processes** in respect of the **Third Party Applications** from the exclusive Licensed Field of Use in this **Agreement**, and **PHS** shall be free to grant a license to the **Third Party Applicant** under the **Licensed Patent Rights** in respect of the **Third Party Applications**.

## 10. PERFORMANCE

- 10.1 **Licensee** shall use its reasonable commercial efforts to bring the **Licensed Products** and **Licensed Processes** to **Practical Application**. "Reasonable commercial efforts" for the purposes of this provision shall include adherence to the **Commercial Development Plan** in Appendix E and performance of the **Benchmarks** in Appendix D. The efforts of a sublicensee shall be considered the efforts of **Licensee**.
- 10.2 Upon the **First Commercial Sale**, until the expiration or termination of this **Agreement**, **Licensee** shall use its reasonable commercial efforts to make **Licensed Products** and **Licensed Processes** reasonably accessible to the United States public.
- 10.3 **Licensee** agrees, after its **First Commercial Sale**, to make reasonable quantities of **Licensed Products** or materials produced through the use of **Licensed Processes** available to patient assistance programs at cost. Patient assistance programs are programs run by pharmaceutical companies to provide free medications to people who cannot afford to buy their medicine. For each indication in each calendar year, the quantity of **Licensed Products** to be made available under this provision available to patient assistance programs at cost shall be defined as the higher of: (i) the maximum quantity of **Licensed Products** for such indication that was available in the previous calendar year (whether or not such **Licensed Products** were actually supplied); and (ii) [\*\*] percent of the total number of **Licensed Products** for such indication prescribed within the United States and its dependant territories in the previous calendar year.
- 10.4 **Licensee** agrees, after its **First Commercial Sale** in a country in the **Licensed Territory** and as part of its marketing and product promotion in such country, to develop educational materials (e.g., brochures, website, etc.) directed to patients and physicians in that country detailing the **Licensed Products** or medical aspects of the prophylactic and therapeutic uses of the **Licensed Products** to the extent permitted by law in such country.
- 10.5 **Licensee** agrees to supply, upon request, to the Mailing Address for **Agreement** Notices indicated on the Signature Page, the Office of Technology Transfer, **NIH** with inert samples of the **Licensed Products** or **Licensed Processes** or their packaging for educational and display purposes only

## 11. INFRINGEMENT AND PATENT ENFORCEMENT

- 11.1 **PHS** and **Licensee** agree to notify each other promptly of each infringement or possible infringement of the **Licensed Patent Rights**, as well as, any facts which may materially affect the validity, scope, or enforceability of the **Licensed Patent Rights** of which either party becomes aware.

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- 11.2 Pursuant to this **Agreement** and the provisions of 35 U.S.C. Part 29, **Licensee** may in accordance with the provisions of Paragraph 11.3:

- (a) bring suit in its own name, at its own expense, and on its own behalf for infringement of presumably valid claims in the **Licensed Patent Rights**;
- (b) in any suit, enjoin infringement and collect for its use, damages, profits, and awards of whatever nature recoverable for the infringement; or
- (c) settle any claim or suit for infringement of the **Licensed Patent Rights** .provided, however, that **PHS** and appropriate Government authorities shall have the first right to take such actions.

- 11.3 If **Licensee** desires to initiate a suit for patent infringement, **Licensee** shall notify **PHS** in writing. If **PHS** does not notify **Licensee** of its intent to pursue legal action within [\*\*] days, **Licensee** shall be free to initiate suit. **PHS** shall have a continuing right to intervene in the suit. **Licensee** shall take no action to compel the **Government** either to initiate or to join in any suit for patent infringement. **Licensee** may request the **Government** to initiate or join in any suit if necessary to avoid dismissal of the suit Should the **Government** be made a party to any suit by motion or any other action of **Licensee**, **Licensee** shall reimburse the **Government** for any costs, expenses, or fees which the Government incurs as a result of the motion or other action. In all cases, **Licensee** agrees to keep **PHS** reasonably apprised of the status and progress of any litigation. Before **Licensee** commences an infringement action, **Licensee** shall notify **PHS** and give careful consideration to the views of **PHS** and to any potential effects of the litigation on the public health in deciding whether to bring suit.

- 11.4 In the event that a declaratory judgment action alleging invalidity or non-infringement of any of the **Licensed Patent Rights** shall be brought against **Licensee** or raised by way of counterclaim or affirmative defense in an infringement suit brought by **Licensee** under Paragraph 11.3, pursuant to this **Agreement** and the provisions of 35 U.S.C. Part 29 or other statutes, **Licensee** may:

- (a) defend the suit in its own name, at its own expense, and on its own behalf for presumably valid claims in the **Licensed Patent Rights**;
- (b) in any suit, ultimately to enjoin infringement and to collect for its use, damages, profits, and awards of whatever nature recoverable for the infringement; and
- (c) settle any claim or suit for declaratory judgment involving the **Licensed Patent Rights**-provided, however, that **PHS** and appropriate **Government** authorities shall have the first right to take these actions and shall have a continuing right to intervene in the suit; and
- (d) If **PHS** does not notify **Licensee** of its intent to respond to the legal action within a reasonable time, **Licensee** shall be free to do so. **Licensee** shall take no action to compel the **Government** either to initiate or to join in any declaratory judgment action. **Licensee** may

request the **Government** to initiate or to join any suit if necessary to avoid dismissal of the suit. Should the **Government** be made a party to any suit by motion or any other action of **Licensee**, **Licensee** shall reimburse the **Government** for any costs, expenses, or fees, which the **Government** incurs as a result of the motion or other action. If **Licensee** elects not to defend against the declaratory judgment action, **PHS**, at its option, may do so at its own expense. In all cases, **Licensee** agrees to keep **PHS** reasonably apprised of the status and progress of any litigation. Before **Licensee** commences an infringement action,

**Licensee** shall notify **PHS** and give careful consideration to the views of **PHS** and to any potential effects of the litigation on the public health in deciding whether to bring suit.

- 11.5 In any action under Paragraphs 11.2, 11.3 or 11.4 the expenses including costs, fees, attorney fees, and disbursements, shall be paid by **Licensee**. The value of any recovery made by **Licensee** through court judgment or settlement shall be treated as **Net Sales** and subject to earned royalties.
- 11.6 **PHS** shall cooperate fully with **Licensee** in connection with any action under Paragraphs 11.2, 11.3 or 11.4. **PHS** agrees promptly to provide access to all necessary documents and to render reasonable assistance in response to a request by **Licensee**.

12. **NEGATION OF WARRANTIES AND INDEMNIFICATION**

- 12.1 **PHS** offers no warranties other than those specified in Article 1.
- 12.2 **PHS** does not warrant the validity of the **Licensed Patent Rights** and makes no representations whatsoever with regard to the scope of the **Licensed Patent Rights**, or that the **Licensed Patent Rights** may be exploited without infringing other patents or other intellectual property rights of third parties.
- 12.3 **PHS** MAKES NO WARRANTIES, EXPRESS OR IMPLIED, OF MERCHANTABILITY OR FITNESS FOR A PARTICULAR PURPOSE OF ANY SUBJECT MATTER DEFINED BY THE CLAIMS OF THE **LICENSED PATENT RIGHTS** OR TANGIBLE MATERIALS RELATED THERETO.
- 12.4 **PHS** does not represent that it shall commence legal actions against third parties infringing the **Licensed Patent Rights**.
- 12.5 **Licensee** shall indemnify and hold **PHS**, its employees, students, fellows, agents, and consultants harmless from and against all liability, demands, damages, expenses, and losses, including but not limited to death, personal injury, illness, or property damage in connection with or arising out of:
- (a) the use by or on behalf of **Licensee**, its sublicensees, directors, employees, or third parties of any **Licensed Patent Rights**; or
  - (b) the design, manufacture, distribution, or use of any **Licensed Products**, **Licensed Processes** or materials by **Licensee**, or other products or processes developed in connection with or arising out of the **Licensed Patent Rights**.
- 12.6 **Licensee** agrees to maintain a liability insurance program consistent with sound business practice.

13. **TERM, TERMINATION, AND MODIFICATION OF RIGHTS**

- 13.1 This **Agreement** is effective when signed by all parties, unless the provisions of Paragraph 14.16 are not fulfilled, and shall extend to the expiration of the last to expire of the **Licensed Patent Rights** unless sooner terminated as provided in this Article 13.
- 13.2 In the event that **Licensee** is in default in the performance of any material obligations under this **Agreement**, including but not limited to the obligations listed in Paragraph 13.5, and if the default has not been remedied within [\*\*] days after the date of notice in writing of the default, **PHS** may terminate this **Agreement** by written notice and pursue outstanding royalties owed through procedures provided by the Federal Debt Collection Act.

- 13.3 In the event that **Licensee** becomes insolvent, files a petition in bankruptcy, has such a petition filed against it that is not discharged within ninety (90) days, determines to file a petition in bankruptcy, **Licensee** shall immediately notify **PHS** in writing. Furthermore, **PHS** shall have the right to terminate this **Agreement** immediately upon **Licensee**'s receipt of written notice.
- 13.4 **Licensee** shall have a unilateral right to terminate this **Agreement** or any licenses in any country or territory by giving **PHS** sixty (60) days written notice to that effect.
- 13.5 **PHS** shall specifically have the right to terminate or modify, at its option, this **Agreement**, if **PHS** determines that the **Licensee**:
- (a) is not executing the **Commercial Development Plan** submitted with its request for a license and the **Licensee** cannot otherwise demonstrate to **PHS**' satisfaction that the **Licensee** has taken, or can be expected to take within a reasonable time, effective steps to achieve **Practical Application** of the **Licensed Products** or **Licensed Processes**;
  - (b) has not achieved the **Benchmarks** as may be modified under Paragraph 9.2;
  - (c) has willfully made a false statement of, or willfully omitted a material fact in the license application or in any report required by this **Agreement**;
  - (d) has committed a material breach of a covenant or agreement contained in this **Agreement**;

- (e) is not keeping **Licensed Products** in a commercially reasonable manner available to the public after commercial use commences;
- (f) cannot reasonably satisfy unmet health and safety needs; or
- (g) cannot reasonably justify a failure to comply with the domestic production requirement of Paragraph 5.2 unless waived.

- 13.6 In making the determination referenced in Paragraph 13.5, **PHS** shall take into account (a) the normal course of such commercial development programs relating to gene therapy conducted with sound and reasonable business practices and judgment, (b) regulatory considerations, and the annual reports submitted by **Licensee** under Paragraph 9.2. Prior to invoking termination or modification of this **Agreement** under Paragraph 13.5, **PHS** shall give written notice to **Licensee** providing **Licensee** specific notice of, and a **[\*\*]** day opportunity to respond to, **PHS**' concerns as to the items referenced in 13.5(a)-13.5(g). If **Licensee** fails to alleviate **PHS**' concerns as to the items referenced in 13.5(a)-13.5(g) or fails to initiate corrective action to **PHS**' reasonable satisfaction, **PHS** may terminate this **Agreement**.
- 13.7 When the public health and safety so require, and after written notice to **Licensee** providing **Licensee** a **[\*\*]** day opportunity to respond, **PHS** shall have the right to require **Licensee** to grant sublicenses to responsible applicants, on reasonable terms, in any **Licensed Fields of Use** under the **Licensed Patent Rights**, unless **Licensee** can reasonably demonstrate that the granting of the sublicense would not materially increase the availability to the public of the subject matter of the **Licensed Patent Rights**. **PHS** shall not require the granting of a sublicense unless the responsible applicant has first negotiated in good faith with **Licensee**.
- 13.8 **PHS** reserves the right according to 35 U.S.C. 5209(d)(3) to terminate or modify this **Agreement** if it is determined that this action is necessary to meet the requirements for public use specified by federal regulations issued after the date of the license and these requirements are not reasonably satisfied by **Licensee**.

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- 13.9 Within **[\*\*]** days of receipt of written notice of **PHS**' unilateral decision to modify or terminate this **Agreement**, **Licensee** may, consistent with the provisions of 37 C.F.R. §404.11, appeal the decision by written submission to the designated **PHS** official. The decision of the designated **PHS** official shall be the final agency decision. **Licensee** may thereafter exercise any and all administrative or judicial remedies that may be available.
- 13.10 Within **[\*\*]** days of expiration or termination of this **Agreement** under this Article 13, a final report shall be submitted by **Licensee**. Any royalty payments, including those incurred but not yet paid (such as the full minimum annual royalty), and those related to patent expense, due to **PHS** shall become immediately due and payable upon termination or expiration. If terminated under this Article 13, sublicensees may elect to convert their sublicenses to direct licenses with **PHS** pursuant to Paragraph 4.3. Unless otherwise specifically provided for under this **Agreement**, upon termination or expiration of this **Agreement**, **Licensee** shall return all **Licensed Products** or other materials included within the **Licensed Patent Rights** to **PHS** or provide **PHS** with certification of the destruction thereof. **Licensee** may not be granted additional **PHS** licenses if the final reporting requirement is not fulfilled.

#### 14. GENERAL PROVISIONS

- 14.1 Neither party may waive or release any of its rights or interests in this **Agreement** except in writing. The failure of a party to assert a right hereunder or to insist upon compliance with any term or condition of this **Agreement** shall not constitute a waiver of that right by that party or excuse a similar subsequent failure to perform any of these terms or conditions by the other party.
- 14.2 This **Agreement** constitutes the entire agreement between the parties relating to the subject matter of the **Licensed Patent Rights**, **Licensed Products** and **Licensed Processes**, and all prior negotiations, representations, agreements, and understandings are merged into, extinguished by, and completely expressed by this **Agreement**.
- 14.3 The provisions of this **Agreement** are severable, and in the event that any provision of this **Agreement** shall be determined to be invalid or unenforceable under any controlling body of law, this determination shall not in any way affect the validity or enforceability of the remaining provisions of this **Agreement**.
- 14.4 If either party desires a modification to this **Agreement**, the parties shall, upon reasonable notice of the proposed modification by the party desiring the change, confer in good faith to determine the desirability of the modification. No modification shall be effective until a written amendment is signed by the signatories to this **Agreement** or their designees.
- 14.5 The construction, validity, performance, and effect of this **Agreement** shall be governed by Federal law as applied by the Federal courts in the District of Columbia.
- 14.6 All **Agreement** notices required or permitted by this **Agreement** shall be given by prepaid, first class, registered or certified mail or by an express/overnight delivery service provided by a commercial carrier, properly addressed to the other party at the address designated on the following Signature Page, or to another address as may be designated in writing by the other party. **Agreement** notices shall be considered timely if the notices are received on or before the established deadline date or sent on or before the deadline date as verifiable by Postal Service postmark or dated receipt from a commercial carrier. Parties should request a legibly dated Postal Service postmark or obtain a dated receipt from a commercial carrier or the Postal Service. Private metered postmarks shall not be acceptable as proof of timely mailing. Notices can also be sent by an email, or a fax.

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- 14.7 This **Agreement** shall not be assigned or otherwise transferred (including any transfer by legal process or by operation of law, and any transfer in bankruptcy or insolvency, or in any other compulsory procedure or order of court) except to **Licensee's Affiliate(s)** without the prior written consent of **PHS**. The parties agree that the identity of the parties is material to the formation of this **Agreement** and that the obligations under this **Agreement** are nondelegable. In the event that **PHS** approves a proposed assignment, **Licensee** shall pay **PHS**, as an additional royalty,

[\*\*] percent ([\*\*]%) of the fair market value of any consideration received for any assignment of this **Agreement** within [\*\*] days of the assignment.

- 14.8 **Licensee** agrees in its use of any **PHS**-supplied materials to comply with all applicable statutes, regulations, and guidelines, including **PHS** and **HHS** regulations and guidelines. **Licensee** agrees not to use the materials for research involving human subjects or clinical trials in the United States without complying with 21 C.F.R. Part 50 and 45 C.F.R. Part 46. **Licensee** agrees not to use the materials for research involving human subjects or clinical trials outside of the United States without notifying **PHS**, in writing, of the research or trials and complying with the applicable regulations of the appropriate national control authorities. Written notification to **PHS** of research involving human subjects or clinical trials outside of the United States shall be given no later than [\*\*] days prior to commencement of the research or trials.
- 14.9 **Licensee** acknowledges that it is subject to and agrees to abide by the United States laws and regulations (including the Export Administration Act of 1979 and Arms Export Control Act) controlling the export of technical data, computer software, laboratory prototypes, biological material, and other commodities. The transfer of these items may require a license from the appropriate agency of the U.S. Government or written assurances by **Licensee** that it shall not export these items to certain foreign countries without prior approval of this agency. **PHS** neither represents that a license is or is not required or that, if required, it shall be issued.
- 14.10 To the extent practicable and allowed by law and regulation, **Licensee** agrees to mark the **Licensed Products** or their packaging sold in the United States with all applicable U.S. patent numbers and similarly to indicate "Patent Pending" status. All **Licensed Products** manufactured in, shipped to, or sold in other countries shall be, to the extent practicable and allowed by law and regulation in such countries, marked in a manner to preserve **PHS** patent rights in those countries.
- 14.11 By entering into this **Agreement**, **PHS** does not directly or indirectly endorse any product or service provided, or to be provided, by **Licensee** whether directly or indirectly related to this **Agreement**. **Licensee** shall not state or imply that this **Agreement** is an endorsement by the Government, **PHS**, any other Government organizational unit, or any Government employee. Additionally, **Licensee** shall not use the names of **NIH**, **FDA**, **PHS**, or **HHS** or the Government or their employees in any advertising, promotional, or sales literature without the prior written approval of **PHS**.
- 14.12 The parties agree to attempt to settle amicably any controversy or claim arising under this **Agreement** or a breach of this **Agreement**, except for appeals of modifications or termination decisions provided for in Article 13. **Licensee** agrees first to appeal any unsettled claims or controversies to the designated **PHS** official, or designee, whose decision shall be considered the final agency decision. Thereafter, **Licensee** may exercise any administrative or judicial remedies that may be available.
- 14.13 Nothing relating to the grant of a license, nor the grant itself, shall be construed to confer upon any person any immunity from or defenses under the antitrust laws or from a charge of patent misuse, and the acquisition and use of rights pursuant to 37 C.F.R. Part 404 shall not be immunized from the operation of state or Federal law by reason of the source of the grant.

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- 14.14 Any formal recordation of this **Agreement** required by the laws of any **Licensed Territory** as a prerequisite to enforceability of the **Agreement** in the courts of any foreign jurisdiction or for other reasons shall be carried out by **Licensee** at its expense, and appropriately verified proof of recordation shall be promptly furnished to **PHS**.
- 14.15 Paragraphs 4.3, 8.1, 9.5-9.7 (in respects of sales carried out prior to termination), 12.1-12.4, 12.5 (in respects of acts carried out prior to termination), 13.9, 13.10, 14.12 and 14.15 of this **Agreement** shall survive termination of this **Agreement**.
- 14.16 The terms and conditions of this **Agreement** shall, at **PHS**' sole option, be considered by **PHS** to be withdrawn from **Licensee**'s consideration and the terms and conditions of this **Agreement**, and the **Agreement** itself to be null and void, unless this **Agreement** is executed by the **Licensee** and a fully executed original is received by **PHS** within [\*\*] days from the date of **PHS** signature found at the Signature Page.

SIGNATURES BEGIN ON NEXT PAGE

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## PHS PATENT LICENSE AGREEMENT - EXCLUSIVE

### SIGNATURE PAGE

For **PHS**:

/s/ Richard U. Rodriquez  
Richard U. Rodriguez  
Director, Division of Technology Development and Transfer  
Office of Technology Transfer  
National Institutes of Health

8-5-11  
Date

Mailing Address or E-mail Address for **Agreement** notices and reports:

Chief, Monitoring & Enforcement Branch  
Office of Technology Transfer  
National Institutes of Health  
6011 Executive Boulevard, Suite 325  
Rockville, Maryland 20852-3804 U.S.A.

E-mail: LicenseNotices\_Reports@mail.nih.gov



For **Licensee** (Upon, information and belief, the undersigned expressly certifies or affirms that the contents of any statements of **Licensee** made or referred to in this document are truthful and accurate.)

By:

/s/ Piers Morgan

10 August 2011

Signature of Authorized Official

Date

Piers Morgan

Chief Financial Officer

Amsterdam Molecular Therapeutics

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Mailing Address for **Agreement** notices:

Chief Executive Officer

Amsterdam Molecular Therapeutics

P.O. Box 22506

1100 DA Amsterdam

The Netherlands

Tel. +31(0)20 566 7394

I. Official and Mailing Address for Financial notices (**Licensee's** contact person for royalty payments)

Piers Morgan

Chief Financial Officer

Amsterdam Molecular Therapeutics

P.O. Box 22506

1100 DA Amsterdam

The Netherlands

Tel.+31(0)20 566 7394

E-mail: p.morgan@amtbiopharma.com

Any false or misleading statements made, presented, or submitted to the **Government**, including any relevant omissions, under this **Agreement** and during the course of negotiation of this **Agreement** are subject to all applicable civil and criminal statutes including Federal statutes 31 U.S.C. §§3801-3812 (civil liability) and 18 U.S.C. §1001 (criminal liability including fine(s) or imprisonment).

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**APPENDIX A - PATENT(S) OR PATENT APPLICATION(S)**

**Patent(s) or Patent Application(s):**

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**APPENDIX B - LICENSED FIELDS OF USE AND TERRITORY**

**I. Licensed Fields of Use:**

- (a) Exclusive **Licensed Field of Use:** (i) Use of the **Licensed Patent Rights** for the development and sale of AAV5 based therapeutic products to be delivered to the brain or liver for treatment of human diseases originating in the brain or liver; (ii) Note that arthritis related diseases are expressly excluded.

- (b) Non-Exclusive **Licensed Field of Use:** Use of the **Licensed Patent Rights** for the development and sale of AAV5 based therapeutic products to treat human diseases other than the ones covered under (a)(i).

II. **Licensed Territory:**

- (a) Worldwide.

**APPENDIX C - ROYALTIES**

**Royalties:**

- I. **Licensee** agrees to pay to **PHS** a noncreditable, nonrefundable license issue royalty in the amount of one hundred forty thousand dollars (\$140,000). Payment will be made in two tranches, the first payment of [\*\*] dollars (\$[\*\*]) being payable within [\*\*] days from the effective date of this **Agreement**; the second payment of [\*\*] dollars (\$[\*\*]) being payable on March 1, 2012. If this **Agreement** is terminated by **Licensee** on or before March 1, 2012, **Licensee** agrees to pay the remaining tranche of license issue royalty in full within [\*\*] days before termination
- II. **Licensee** agrees to pay to **PHS** a nonrefundable minimum annual royalty in the amount of [\*\*] dollars (\$[\*\*]) as follows:
- (a) The first minimum annual royalty is due within [\*\*] days of the effective date of this **Agreement** and may be prorated according to the fraction of the calendar year remaining between the effective date of this **Agreement** and the next subsequent January 1; and
- (b) Subsequent minimum annual royalty payments are due and payable on January 1 of each calendar year and may be credited against any earned royalties due for sales made in that year.
- III. **Licensee** agrees to pay **PHS** earned royalties of [\*\*] percent ([\*\*]%) on **Net Sales** by or on behalf of **Licensee** and its sublicensees.
- IV. **Licensee** agrees to pay **PHS Benchmark** royalties within [\*\*] days of achieving each Benchmark :
- (a) [\*\*] U.S. dollars (\$[\*\*]) - Initiation of each Phase I clinical trial or foreign equivalent.
- (b) [\*\*] U.S. dollars (\$[\*\*]) - Initiation of each Phase II clinical trial or foreign equivalent.
- (c) [\*\*] U.S. dollars (\$[\*\*]) - Initiation of each Phase III clinical trial or foreign equivalent.
- (d) Initiation of first Marketing Approval or foreign equivalent for any indications in the liver in the following jurisdictions/countries:
- [\*\*]
- (e) Initiation of first Marketing Approval or foreign equivalent for any indications in the brain in the following jurisdictions/countries:
- [\*\*]
- V. **Licensee** agrees to pay **PHS** additional sublicensing royalties, as following, on the fair market value of any consideration received for granting each sublicense within [\*\*] days of the execution of each sublicense:
- (a) For any sublicense executed by the **Licensee** before the [\*\*], **Licensee** agrees to pay a sublicensing royalty of [\*\*] percent ([\*\*]%) and
- (b) For any sublicense executed by the **Licensee** after the [\*\*], **Licensee** agrees to pay a sublicensing royalty of [\*\*] percent ([\*\*]%) and
- (c) For any sublicense executed by the **Licensee** either [\*\*], whichever comes first. **Licensee** agrees to pay a sublicensing royalty of [\*\*] percent ([\*\*]%).

Contractual payments made by a sublicensee to the **Licensee** or an Affiliate received after the effective date of this **Agreement** for costs, services and expenses for the **Licensee** or Affiliate to conduct, supervise or participate in one or more clinical trial(s) for the development of the **Licensed Products** shall not be accounted for as sublicensing royalties.

**APPENDIX D - BENCHMARKS AND PERFORMANCE**

**Licensee** agrees to the following **Benchmarks** for its performance under this **Agreement** and, within [\*\*] days of achieving a **Benchmark**, shall notify **PHS** that the **Benchmark** has been achieved.

**Benchmarks for Licensed Products of Orphan Indication (there is no formal Phase III clinical trial required for Marketing Approval) - liver**

[\*\*]

**Benchmarks for Licensed Products - brain**

## APPENDIX E - COMMERCIAL DEVELOPMENT PLAN

### Project Plan Details - Liver:

Acute intermittent porphyria (AIP) is an autosomal dominant inherited condition caused by mutations in the porphobilinogen deaminase (PBGD) gene. The PBGD gene is located on chromosome 11q24.1-24.2 and spread over fifteen exons. The protein encoded by this gene is a rate-limiting enzyme, the PBGD enzyme, in the haem synthetic pathway.

More than 225 mutations of the PBGD gene have been described, all of them associated with loss of catalytic function. The disease shows incomplete penetrance and only 20-50% of persons with one or more of the described mutations exhibit clinical symptoms of the disease. The genetic disorder results in a 50% reduction of PBGD enzymatic activity. This reduction of hepatic PBGD activity leads to an accumulation of toxic metabolites resulting from the blockade within the haem synthesis pathway. Concentrations of haem precursors porphobilinogen (PBG) and delta-aminolevulinic acid (ALA) increase in blood and urine. Lack of haem and/or accumulation of these metabolites are responsible for the acute attacks characteristic of this disease (Kauppinen et al 2005; Herrick and McColl 2005). Currently, there is no treatment available for the disease.

Over the last couple of years **Licensee** has explored AMT-021 (replication defective recombinant adeno-associated viral vector, AAV, containing the porphobilinogen deaminase gene) for therapeutic intervention in AIP. AMT-021 is an AAV with pseudotype 5 capsid, which expresses the human PBGD gene under the transcriptional control of a liver specific promoter. The therapeutic expression cassette consists of the human PBGD cDNA (codon optimised for human expression) inserted downstream of the liver specific promoter EalbAAT and upstream of a human PBGD polyadenylation sequence.

AMT-021 acts by delivering the PBGD expression cassette directly into hepatocytes. The increase of PBGD enzymatic activity in the liver of AIP patients will provide sufficient enzyme to prevent the accumulation of toxic metabolites and thus, prevent porphyric attacks.

The aim of the project is to bring AAV5-PBDG therapy to patients. **Licensee** has already secured orphan designation for AAV5-PBDG treatment for AIP in Europe. The table below describes the outline development plans, starting from a research batch production, and moving through to primate proof-of-concept, tox batch, pre-observational study, product development, GMP production, Phase I/II clinical trial, Phase II/III clinical trial, all the way to regulatory filing. Please note that the timelines are preliminary only, and that it is the nature of scientific and clinical development that planned timelines may change.

The aim of this project is to develop a gene therapy product for the treatment of AIP, and to deliver a data package that is suitable for the submission and approval by the European and North American regulatory authorities.

### Vector development and manufacturing

To develop a gene therapy for PBGD deficient patients, AAV5-PBDG product was designed to express the human PBGD gene under the control of a liver specific promoter. AAV5-PBDG was produced in insect cells using the recombinant baculovirus method; sufficient amount of material was produced for efficacy studies in mice. Methods to determine the quantity and purity of the rAAV batches were developed. A purification process including chromatography and filtration steps was developed, further optimization and characterization of the scale-up procedure will be performed before a final batch for toxicology, for proof of principle and for clinical trials can be produced.

### PoC in pre-clinical models

Because total deficiency of PBGD is lethal in mice, a compound heterozygous mouse (PBGD<sup>+/-</sup> referred to as AIP mice) with ~35% of normal hepatic PBGD activity, has been developed as an established model to study AIP. This murine model of AIP exhibits, after disease induction with phenobarbital (Pb), the typical biochemical characteristics

of human AIP, notably, decreased hepatic PBGD activity, massively increased urinary excretion of haem precursors (ALA and PBG) and decreased motor function.

AIP mice were used to test the AAV5-PBDG product. The therapeutic effect was evaluated three months after a single intravenous administration of AAV5-PBDG. Efficacy of the therapy was demonstrated as the treatment was able to prevent disease induction with Pb. ALA and PBG levels in treated animals was reduced, and motor disturbance induced by Pb treatment, as measured in the Rotarod test, was almost completely abolished. In addition, PBGD enzymatic activity increased in the AAV5-PBDG treated group 10 times over that of the control group.

This initial PoC will be repeated with the final version of the therapeutic vector following the completion of the vector development and manufacturing optimization. The final PoC will include the following:

### PoC in rodent disease model

- PoC in non-human primates, based on agreed protocol

### GLP Toxicology

The aim of this section is to deliver toxicology study report suitable for the submission to the regulatory authority. The work will entail the following:

- Scientific advice from a regulatory body (AEMPS and/or EMA) for safety and toxicology package
- GLP toxicology study in rodents rats or mice, including any required biodistribution studies
- Supportive data for toxicology study in non-human primates
- GLP germline transmission study

Toxicology study design will take into account:

- Identification of potential target organs of biological activity and of potential target organs of toxicity

- Eventual concomitant medication (e.g. immunosuppressants, standard co-medication)
- Environmental risk/shedding
- Analysis of appropriateness of surrogate markers of efficacy/safety
- Any other relevant issues as may be identified

#### Clinical observational, pre-intervention study/studies

Before entering the interventional clinical study, an observation clinical study will be conducted to provide baseline information on the course of the disease by recording episodes AIP, abdominal pain, hospitalizations, extent of any possible known or unknown to be related to AIP symptomatology, incidence of (adverse) clinical events per year, etc. Sufficient data will be collected to provide a clinical picture to obtain a baseline data and to determine how efficacy will be shown during the interventional clinical trial.

#### Phase I/II

The clinical phase I/II should include an estimated minimum [\*\*] patients that are administered the gene therapy drug, and are followed up and clinically assessed for at least [\*\*] months following drug administration. The primary aim of the clinical study will be safety and efficacy of the AAV5-PBDG product. The clinical trial will include all biochemical, imaging, clinical and functional assays to assess the disease state and change therein over time, the phenotypic disease variation, as well as the overall clinical and psychosocial or other health status or change therein over time of the individual trial subjects, both before, during and following drug administration.

#### Phase II/III & Regulatory submission

After successful completion of Phase I/II study a Phase II/III trial will be conducted with the aim of bringing the AIP therapy to market. **Licensee** estimates that [\*\*] patients in total would be sufficient for regulatory filing of this product, as AIP is an ultra-orphan disease with a very limited patient number world-wide.

#### Project Plan Details-Brain (Parkinson's Disease)

Parkinson's disease (PD) is a progressive neurodegenerative disease, resulting in tremors, stiffness, slowness of movement, and lack of coordination. Patients are faced with a severely debilitating disease and a serious loss in quality of life. PD is caused by degeneration and death of nerve cells in a specific part of the brain known as the substantia nigra. These cells produce dopamine, a substance necessary for communication between nerve cells involved in the coordination of movement.

PD is the second most common neurodegenerative disease. It usually affects people over 65, with an estimated total of 4.5 million patients worldwide. Due to increasing life expectancy of the general population, the number of patients with PD is expected to double to around 9 million patients between now and 2030.

An ideal therapy for PD would decrease disability and slow down or halt disease progression. Unfortunately, such treatments are not available yet and current therapies are limited to symptomatic treatment only. These include levodopa, dopamine agonists, monoamine oxidase B (MAO-B) inhibitors and anticholinergic agents.

Glial cell line-derived neurotrophic factor (GDNF) was shown to promote the survival and differentiation of dopaminergic neurons. The therapy aims to protect and enhance the function of the dopamine-producing nerve cells in the brain. To date a number of clinical trials have been conducted in which recombinant GDNF protein has been directly delivered to the PD brain, using a delivery pump device implanted into patients' abdomen. Although the results were inconsistent, due to the difficulty of delivering protein continuously into the brain via an implanted pump, some patients have shown a significant clinical response to the treatment. It is therefore not a question whether this approach works, because it definitely did in some patients, but rather how it can be done more consistently. AAV-GDNF gene therapy treatment would result in continues delivery of GDNF protein into brain, and is therefore likely to result in significant clinical benefit for PD patients.

**Licensee** has recently started preclinical development of AAV-GDNF gene therapy that will introduce the gene coding for GDNF using recombinant adeno associated virus vector (AAV). AAV serotype 5 has been shown to be the serotype of choice for gene delivery into the brain. After successful proof of concept (POC) and toxicology studies in rodents and primates, AMT will start an extensive clinical development.

The aim of this project is to develop a gene therapy product for the treatment of Parkinson's disease, and to deliver a data package that is suitable for the submission and approval by the European and North American regulatory authorities.

#### Vector development and manufacturing

To develop a gene therapy for Parkinson's disease, AAV-GDNF product was designed to expresses the human GDNF and is produced in insect cells using the recombinant baculovirus method. The AAV5-GDNF is based on **Licensee**'s standard manufacturing process, but in addition incorporates recent new technology of the basic process and makes use of an optimized Rep baculovirus construct in the upstream process and an additional chromatography step in the downstream process. This optimisation delivers enhanced quality and robustness of the AAV5-GDNF product. This process is fully scalable and allows for manufacturing of sufficient GMP-compliant product for PD patients.

#### Characterization of AAV5-GDNF

The AAV5-GDNF was tested in a functional in vitro assay in cultured E13.5 rat DRG explants. Vigorous neural outgrowth was observed, indicating that the produced AAV5-GDNF is capable of mediating secretion of biologically functional recombinant GDNF.

#### In vivo characterization

Subsequently, an in-vivo characterisation of the AAV5-GDNF has been conducted. Three different concentrations of AAV5-GDNF were injected unilaterally into the rat striatum. Brains were analyzed for GDNF expression 6 weeks post injection using immunohistochemistry. Resulting data demonstrated that there is a strong, concentration dependent GDNF expression throughout the injected hemisphere.

#### PoC in pre-clinical models

The produced AAV5-GDNF will be used to show biological activity and efficacy in animal models of Parkinson's disease. These experiments will be conducted using rat models of Parkinson's disease (in collaboration with University of Lund, Sweden) as well as non-human primates' model of Parkinson's disease (in

collaboration with CEA, Paris, France). In addition to distribution studies, onset and kinetics of GDNF expression, neurochemical measurements (dopamine and dopamine metabolites), immunohistochemistry and behavioral studies will be conducted to test for functional improvement.

GLP Toxicology

The definitive design of the actual studies will be finalized after discussions with relevant agencies. **Licensee** proposes to conduct a six months study in mice and in parallel a 6-12 months study in non-human primates to account for the safety of the drug. The studies will comprise four test groups: 1. Control (vehicle), 2. Low dose (No observed effect level (NOEL) in the proof-of concept studies), 3. Mid-dose (highest dose considered for clinical studies), and 4. High dose (10 times higher than the mid-dose).

The protocol will include the following evaluations:

- Clinical Signs: recorded daily, beginning 7 days prior to surgery
- Food Consumption: recorded daily, beginning 7 days prior to surgery
- Body Weight: Once pre-surgery, day of surgery, then bi-weekly
- Clinical Chemistry: Twice a month presurgery, one week post surgery, then monthly
- Hematology: Twice a month presurgery, one week post surgery, then monthly
- Coagulation: Twice a month presurgery, one week post surgery, then monthly
- Antibodies against GDNF or AAV5 in plasma, twice prior to surgery, monthly thereafter.
- PK - CSF: To determine if there is GDNF in the CSF, twice prior to surgery, monthly thereafter.
- Neurological Examination: Twice prior to surgery, Day 7 post surgery, monthly thereafter
- MRI (T1,T2): Once prior to surgery, within three hours post surgery, and within three days prior to necropsy.
- Pathology
- 1. Gross pathology at necropsy
- 2. Selected peripheral tissues collected for histopathological analysis by a Board Certified Pathologist
- 3. Complete CNS histopathological assessment by a Board Certified Neuropathologist, peer reviewed by another Board Certified Pathologist
- Q-PCR in selected organs in order to assess any biodistribution of the vector DNA to other organs.

Phase I/II

The primary objective of the clinical phase I/II will be to assess the safety and feasibility of intra-putaminal delivery of AAV5-GDNF to patients with PD. Secondary objectives include measuring clinical efficacy and demonstrating improvement in a surrogate marker end point (18F-Dopa PET) as proof of concept.

**Licensee** is proposing a single centre open label trial of striatally delivered AAV5-GDNF in PD employing a dose escalation design to assess the mentioned primary and secondary outcome measures. **Licensee** anticipates enrolling [\*\*] patients in this study, with an escalating dose group design with [\*\*] patients in each dose group. **Licensee** will start with the lowest dose and progress in an incremental way to higher doses.

Primary outcome assessments will be performed at [\*\*] post intra-putaminal infusion of AAV5-GDNF. Clinical secondary outcome assessments will be performed at [\*\*] post intra-putaminal infusion of AAV5-GDNF. 18F-dopa PET secondary outcome assessments will be performed at [\*\*] months and [\*\*] months post intra-putaminal infusion of AAV5-GDNF.

If feasibility and safety is confirmed and, serial PET imaging demonstrates increased 18F-dopa uptake with a trend towards clinical improvement, we will proceed to phase 2/3 clinical trials.

Phase II/III, Phase III & Regulatory submission

After successful completion of Phase I/II study, two additional clinical trials will be required. The final plans for these trials will be optimized based on the outcome of the Phase I/II study. **Licensee** estimates [\*\*] patients to be enrolled in the Phase II/III clinical study, and [\*\*] patients to be enrolled in the pivotal trial, the details however will be established, based on the outcome of the Phase I/II trial.

Additional indication;

In addition to the above, **Licensee** has an active programs in hemophilia B using AAV5-Factor IX, in hemophilia A using AAV5-Factor VIII, in Sanfilippo B - currently conducted by Institut Pasteur, using AAV5-NaGlu gene, and a program for the development of treatment for Usher syndrome type 1 (USH1) using AAV5-MY07A. Additional early stage programs are under evaluation.

**APPENDIX F - EXAMPLE ROYALTY REPORT**

**Required royalty report information includes:**

- OTT license reference number (L-XXX-200X/0)
- Reporting period
- Catalog number and units sold of each Licensed Product (domestic and foreign)
- Gross Sales per catalog number per country
- Total Gross Sales
- Itemized deductions from Gross Sales
- Total Net Sales
- Earned Royalty Rate and associated calculations
- Gross Earned Royalty
- Adjustments for Minimum Annual Royalty (MAR) and other creditable payments made
- Net Earned Royalty due

**Example**

Catalog Number	Product Name	Country	Units Sold	Gross Sales (US\$)
1	A	【**】	【**】	【**】
1	A	【**】	【**】	【**】
1	A	【**】	【**】	【**】
2	B	【**】	【**】	【**】
3	C	【**】	【**】	【**】
4	D	【**】	【**】	【**】
Total Gross Sales				【**】
Less Deductions:				
Freight				【**】
Returns				【**】
Total Net Sales				【**】
Royalty Rate				【**】
Royalty Due				【**】
Less Creditable Payments				【**】
Net Royalty Due				【**】

### **APPENDIX G - ROYALTY PAYMENT OPTIONS**

The OTT License Number MUST appear on payments, reports and correspondence.

#### **Automated Clearing House (ACH) for payments through U.S. banks only**

The NIH encourages our licensees to submit electronic funds transfer payments through the Automated Clearing House (ACH). Submit your ACH payment through the U.S. Treasury web site located at: **<https://www.pay.gov>**. Locate the “NIH Agency Form” through the Pay.gov “Agency List”.

#### **Electronic Funds Wire Transfers**

The following account information is provided for wire payments. In order to process payment via Electronic Funds Wire Transfer sender MUST supply the following information within the transmission:

Drawn on a **U.S. bank account** via FEDWIRE should be sent directly to the following account:

Beneficiary Account:	Federal Reserve Bank of New York or TREAS NYC
Bank:	Federal Reserve Bank of New York
ABA#	021030004
Account Number:	75080031
Bank Address:	33 Liberty Street, New York, NY 10045
Payment Details:	License Number (L-XXX-XXXX)
	Name of Licensee

Drawn on a **foreign bank account** should be sent directly to the following account. Payment must be sent in **U.S. Dollars (USD)** using the following instructions:

Beneficiary Account:	Federal Reserve Bank of New York/ITS or FRBNY/ITS
Bank:	Citibank N.A. (New York)
SWIFT Code:	CITIUS33
Account Number:	36838868
Bank Address:	388 Greenwich Street, New York, NY 10013
Payment Details (Line 70):	NIH 75080031
	License Number (L-XXX-XXXX)
	Name of Licensee
Detail of Charges (line 71a):	Charge Our

#### **Checks**

All checks should be made payable to “NIH Patent Licensing”

Checks drawn on a **U.S. bank account** and sent by US Postal Service should be sent directly to the following address:

National Institutes of Health (NIH)  
P.O. Box 979071  
St. Louis, MO 63197-9000

Checks drawn on a U.S. bank account and sent by **overnight or courier** should be sent to the following address:

US Bank  
Government Lockbox SL-MO-C2GL  
1005 Convention Plaza  
St. Louis, MO 63101

Checks drawn on a **foreign bank account** should be sent directly to the following address:

National Institutes of Health (NIH)  
Office of Technology Transfer  
Royalties Administration Unit  
6011 Executive Boulevard  
Suite 325, MSC 7660  
Rockville, Maryland 20852

**NATIONAL INSTITUTES OF HEALTH**

**FIRST AMENDMENT TO L-116-2011/0**

This is the first amendment ("**First Amendment**") of the agreement by and between the National Institutes of Health ("**NIH**") within the Department of Health and Human Services ("**HHS**"), and UniQure biopharma B.V. (formerly Amsterdam Molecular Therapeutics N.V. (AMT)) having an effective date of August 10, 2011 and having **NIH** Reference Number L-116-2011/0 ("**Agreement**"). This **First Amendment**, having **NIH** Reference Number L-116-2011/1, is made between the **NIH** through the Office of Technology Transfer, **NIH**, having an address at 6011 Executive Boulevard, Suite 325, Rockville, Maryland 20852-3804, U.S.A., and UniQure biopharma B.V. (formerly Amsterdam Molecular Therapeutics N.V. (AMT)), having an office at Meibergdreef 61, 1105 BA Amsterdam, The Netherlands ("**Licensee**"). This **First Amendment** includes, in addition to the amendments made below, 1) a Signature Page, and 2) Attachment 1 (Royalty Payment Information).

WHEREAS, **NIH** and **Licensee** desire that the **Agreement** be amended a first time as set forth below in order to

- a) Change the name of **Licensee** from Amsterdam Molecular Therapeutics N.V. (AMT) to UniQure biopharma B.V. (UniQure). This name change is the result of a transaction that took place on 30 March 2012, whereby AMT, a public company, was liquidated and all its operations and stocks were transferred to UniQure, a privately held company.
- b) Modify language related to financial terms associated with sublicensing, so as to cause a reduction in financial obligations due to **NIH** from sublicensing of the **Agreement** by **Licensee** in order to expedite the development of therapeutics for rare diseases.

NOW, THEREFORE, in consideration of the mutual covenants and promises contained herein, **NIH** and **Licensee**, intending to be bound, hereby mutually agree to the following:

- 1)
  - a) In Cover page following the list of "licensed patent and patent application", the name of **Licensee** has been changed to UniQure biopharma B.V.
  - b) In the signature page under "signature of authorized official", under "mailing address for **Agreement** notices", and under "official and mailing address for financial notices" "Amsterdam Molecular Therapeutics, N.V." has been changed to UniQure biopharma B.V.
  - c) In the caption of the **Agreement** AMT is changed to UniQure.
- 2) Replace Paragraph 6.7 with the following:  
  
6.7 No multiple royalties shall be payable if any **Licensed Products** or **Licensed Processes** are covered by more than one of the **Licensed Patent Rights**. In the event that this **Agreement** and **NIH** license L-107-2007/0 as amended from time to time apply to the same product sold by the **Licensee** or its sublicensees, then the **Licensee** shall only pay earned royalties, benchmark royalties, and sublicensing royalties under this **Agreement**.
- 3) Replace Appendix C Section V with the following:

**Licensee** agrees to pay **NIH** additional sublicensing royalties, as follows, on the fair market value of any consideration received for granting each sublicense within **[\*\*]** days of the execution of each sublicense:

- (i) For any sublicense executed by the **Licensee** before **[\*\*]**, **Licensee** agrees to pay a sublicensing royalty as in the following formula:

A-267-2012

**CONFIDENTIAL**

Sublicensing Royalty = **[\*\*]**

for the purposes of calculating sublicensing royalties in (i), where  $P/(P+T+L)$  is a fraction in which P represents the **NIH's Licensed Patent Right**, T represents the Intellectual Property (IP) licensed by Licensee from a third party, and where such an IP is related only to an active component of the **Licensed Products** (i.e. gene of interest incorporated into the AAV construct), and L represents **Licensee's** own IP used to make the **Licensed Product**. Furthermore P, T and L, when present, each carries a value of 1.

The value of the fraction  $P/(P+T+L)$  as applied to (i) can never go below **[\*\*]**, and therefore the Sublicensing Royalty as applied to (i) will never go below **[\*\*]**.

(ii) For any sublicense executed by the **Licensee** after [\*\*], Licensee agrees to pay a sublicensing royalty as in the following formula:

$$\text{Sublicensing Royalty} = [**]$$

The value of the fraction  $P/(P+T+L)$  as applied to (ii) can never go below [\*\*], and therefore the Sublicensing Royalty as applied to (ii) will never go below [\*\*]

(iii) For any sublicense executed by the **Licensee** either [\*\*], **Licensee** agrees to pay a sublicensing royalty as in the following formula:

$$\text{Sublicensing Royalty} = [**]$$

The value of the fraction  $P/(P+T+L)$  as applied to (iii) can never go below [\*\*], and therefore the Sublicensing Royalty as applied to (iii) will never go below [\*\*]

Contractual payments made by a sublicensee to the **Licensee** or an **Affiliate** received after the effective date of this **Agreement** for costs, services and expenses for the **Licensee** or **Affiliate** to perform research and development activities, or to conduct, supervise or participate in one or more clinical trial(s) for the development of the **Licensed Products**, or to manufacture clinical and commercial batches of **Licensed Products**, shall not be accounted for in the calculation of sublicensing royalties.

4) **Licensee** shall pay **NIH** an amendment issue royalty in the sum of five hundred thousand US Dollars (\$500,000.00) as follows:

- i) Two hundred and fifty thousand Dollars (\$250,000) shall be paid by **Licensee** within [\*\*] days of the effective date of this **First Amendment**.
- ii) The remaining amount of two hundred and fifty thousand Dollars (\$250,000) shall be paid to **NIH** upon execution by **Licensee** of any new sublicensing or partnership agreement or on the first anniversary of this **First Amendment**, whichever occurs first.

5) In the event any provision(s) of the **Agreement** is/are inconsistent with Attachment 1, such provision(s) is/are hereby amended to the extent required to avoid such inconsistency and to give effect to payment information in such Attachment 1.

6) All terms and conditions of the **Agreement** not herein amended remain binding and in effect.

7) The terms and conditions of this **First Amendment** shall, at **NIH**' sole option, be considered by **NIH** to be withdrawn from **Licensee's** consideration and the terms and conditions of this **First Amendment**, and the **First Amendment** itself, to be null and void, unless this **First Amendment** is executed by **Licensee** and a fully executed original is received by **NIH** within [\*\*] days from the date of **NIH** signature found at the Signature Page.

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8) This **First Amendment** is effective on \_\_\_\_\_ upon execution by all parties.

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#### **FIRST AMENDMENT TO L-116-2011/0**

#### **SIGNATURE PAGE**

In Witness Whereof, the parties have executed this **First Amendment** on the dates set forth below. Any communication or notice to be given shall be forwarded to the respective addresses listed below.

For **NIH**:

/s/ Richard U. Rodriguez  
Richard U. Rodriguez  
Director, Division of Technology Development and Transfer  
Office of Technology Transfer  
National Institutes of Health

5-23-13

Date

Mailing Address or E-mail Address for **Agreement** notices and reports:

Chief, Monitoring & Enforcement Branch, DTD  
Office of Technology Transfer  
National Institutes of Health  
6011 Executive Boulevard, Suite 325  
Rockville, Maryland 20852-3804 U.S.A.

E-mail: LicenseNotices\_Reports@mail.nih.gov

For **Licensee** (Upon information and belief, the undersigned expressly certifies or affirms that the contents of any statements of **Licensee** made or referred to in this document are truthful and accurate.):

/s/ John Alday  
John Alday, CEO UniQurebiopharm B.V.

5-31-13

Date



I. Official and Mailing Address for **Agreement** notices:  
Chief Executive Officer:  
Legal@uniqure.com

II. For invoices, payments, and Financial notices (including royalty payments):  
Finance Dept  
Finance@uniqure.com

uniQure biopharma B.V.  
Meibergdreef 61  
1105BA Amsterdam  
The Netherlands

Phone: 0031 205667394  
Fax: 0031 20 566 9272

Any false or misleading statements made, presented, or submitted to the **Government**, including any relevant omissions, under this **Agreement** and during the course of negotiation of this **Agreement** are subject to all applicable civil and criminal statutes including Federal statutes 31 U.S.C. §§3801-3812 (civil liability) and 18 U.S.C. §1001 (criminal liability including fine(s) or imprisonment).

### **ATTACHMENT 1 - ROYALTY PAYMENT OPTIONS**

The OTT License Number **MUST** appear on payments, reports and correspondence.

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Bank:	Federal Reserve Bank of New York
ABA#	021030004
Account Number:	75080031
Bank Address:	33 Liberty Street, New York, NY 10045
Payment Details:	License Number (L-XXX-XXXX) Name of Licensee

Drawn on a **foreign bank account** should be sent directly to the following account. Payment must be sent in **U.S. Dollars (USD)** using the following instructions:

Beneficiary Account:	Federal Reserve Bank of New York/ITS or FRBNY/ITS
Bank:	Citibank N.A. (New York)
SWIFT Code:	CITIUS33
Account Number:	36838868
Bank Address:	388 Greenwich Street, New York, NY 10013
Payment Details (Line 70):	NIH 75080031 License Number (L-XXX-XXXX) Name of Licensee
Details of Charges (Line 71a):	Charge Our

#### **Checks**

All checks should be made payable to “NIH Patent Licensing”

Checks drawn on a **U.S. bank account** and sent by US Postal Service should be sent directly to the following address:

National Institutes of Health (NIH)  
P.O. Box 979071  
St. Louis, MO 63197-9000

Checks drawn on a U.S. bank account and sent by **overnight or courier** should be sent to the following address:

US Bank  
Government Lockbox SL-MO-C2GL  
1005 Convention Plaza  
St. Louis, MO 63101  
Phone: 314-418-4087

Checks drawn on a **foreign bank account** should be sent directly to the following address:

National Institutes of Health (NIH)  
Office of Technology Transfer  
Royalties Administration Unit  
6011 Executive Boulevard  
Suite 325, MSC 7660  
Rockville, Maryland 20852

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**NATIONAL INSTITUTES OF HEALTH**

**SECOND AMENDMENT TO L-116-2011/0**

This is the second amendment ("**Second Amendment**") of the agreement by and between the National Institutes of Health ("**NIH**") within the Department of Health and Human Services ("**HHS**"), and UniQure biopharma B.V. (formerly Amsterdam Molecular Therapeutics (AMT) B.V.) having an effective date of August 10, 2011 as amended for the first time on May, 31, 2013, and having **NIH** Reference Number L-116-2011/0 and L-116-2011/1 respectively ("**Agreement**"). This **Second Amendment**, having **NIH** Reference Number L-116-2011/2, is made between the **NIH** through the Office of Technology Transfer, **NIH**, having an address at 6011 Executive Boulevard, Suite 325, Rockville, Maryland 20852-3804, U.S.A., and UniQure biopharma B.V. (formerly Amsterdam Molecular Therapeutics (AMT) B.V.), having an office at Meibergdreef 61, 1105 BA Amsterdam, The Netherlands ("**Licensee**"). This **Second Amendment** includes, in addition to the amendments made below, a Signature Page.

WHEREAS, **NIH** and **Licensee** desire that the **Agreement** be amended a second time as set forth below in order to a) clarify the nonexclusive **Field of Use**, b) to update appendices D and E of the **Agreement**, and c) to update Article 6.13 of the **Agreement** with the name of an **Exempt Collaborator** that is approved to work with the **Licensee** on one **Ultra-Orphan Indication**.

NOW, THEREFORE, in consideration of the mutual covenants and promises contained herein, **NIH** and **Licensee**, intending to be bound, hereby mutually agree to the following:

- 1) In Appendix B replace Paragraph I(b) of the **Licensed Field of Use** with the following:
  - (b) Non-Exclusive **Licensed Field of Use**: Use of the **Licensed Patent Rights** for the development and sale of AAV5 based therapeutic products to treat any human disease in any manner, where the treatment of such disease in such manner is not included in the Exclusive **Licensed Field of Use**.
- 2) In Article 6.13 add the following:
  - (h) Institut Pasteur has been approved by the **NIH** as an **Exempt Collaborator** for a clinical work related to Sanfilippo B.
- 3) Replace Appendix D with Appendix D attached to this **Second Amendment** as EXHIBIT 1.
- 4) Replace Appendix E with Appendix E attached to this **Second Amendment** as EXHIBIT 2.
- 5) All terms and conditions of the **Agreement** not herein amended remain binding and in effect.
- 6) The terms and conditions of this **Second Amendment** shall, at **NIH** sole option, be considered by **NIH** to be withdrawn from **Licensee**'s consideration and the terms and conditions of this **Second Amendment**, and the **Second Amendment** itself, to be null and void, unless this **Second Amendment** is executed by **Licensee** and a fully executed original is received by **NIH** within [\*\*] days from the date of **NIH** signature found at the Signature Page.
- 7) This **Second Amendment** is effective on the date of execution by the last party to execute this **Second Amendment**.

A-038-2014

**CONFIDENTIAL**

second Amendment of L-116-2001/0  
Model 09-2006 (updated 8-2010)

[Final] UniQure biopharma, B.V.

October 29, 2013  
L-116-2011/2

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**SECOND AMENDMENT TO L-116-2011/0**

**SIGNATURE PAGE**

In Witness Whereof, the parties have executed this **Second Amendment** on the dates set forth below. Any communication or notice to be given shall be forwarded to the respective addresses listed below.

For **NIH**:

/s/ Richard U. Rodriguez  
Richard U. Rodriguez  
Director, Division of Technology Development and Transfer  
Office of Technology Transfer  
National Institutes of Health

11-6-13  
Date

Mailing Address or E-mail Address for **Agreement** notices and reports:

Chief, Monitoring & Enforcement Branch, DTD  
Office of Technology Transfer  
National Institutes of Health  
6011 Executive Boulevard, Suite 325  
Rockville, Maryland 20852-3804 U.S.A.

E-mail: LicenseNotices\_Reports@mail.nih.gov

For **Licensee** (Upon information and belief, the undersigned expressly certifies or affirms that the contents of any statements of **Licensee** made or referred to in this document are truthful and accurate.):

/s/ Piers J. Morgan P.J. Morgan  
Piers J Morgan, CFO, uniQure biopharma B.V.

November 11, 2013  
Date

I Official and Mailing Address for **Agreement** notices:

Chief Executive Officer;  
*Legal@uniqure.com*

II For invoices, payments, and Financial notices (including royalty payments):

Finance Dept  
*Finance@uniqure.com*

uniQure biopharma B.V.  
Meibergdreef 61  
1105BA Amsterdam  
The Netherlands

Phone: 0031 205667394

Fax: 0031 20 566 9272

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Any false or misleading statements made, presented, or submitted to the **Government**, including any relevant omissions, under this **Agreement** and during the course of negotiation of this **Agreement** are subject to all applicable civil and criminal statutes including Federal statutes 31 U.S.C. §§3801-3812 (civil liability) and 18 U.S.C. §1001 (criminal liability including fine(s) or imprisonment).

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## Exhibit 1

### APPENDIX D — BENCHMARKS AND PERFORMANCE (L-116/2011)

**Licensee** agrees to the following **Benchmarks** for its performance under this **Agreement** and, within **[\*\*]** days after achieving a **Benchmark**, shall notify **PHS** that the **Benchmark** has been achieved.

Note: No formal Phase III clinical trial is required for **Marketing Approval** for any **Orphan Indication**

Benchmarks for a **Licensed Product** of **Orphan Indication** - liver

**[\*\*]**

Benchmarks for a **Licensed Product** - brain

**[\*\*]**

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## Exhibit 2

### APPENDIX E - COMMERCIAL DEVELOPMENT PLAN (L-116-2011)

The table below (table 1) presents a comprehensive list of all uniQure research and development projects utilizing the **Licensed Patent Rights**, according to main disease site and divided into projects that are, **a)** commercial projects, **b)** already in development stages, **c)** active research (there is already internal research activity ongoing and **d)** exploratory research projects (currently being considered as potential projects worth further investigation in the near future).

Table 1: uniQure R&D projects

	Liver (AAV5 based)	Brain & CNS (AAV5 based)
Commercial Projects	[**]	[**]
Development Projects	[**]	[**]
Active Research Projects	[**]	[**]
Exploratory Research Projects	[**]	[**]

Detailed information on the development and active research projects is provided below.

NOTE: All dates contained in this Commercial Development Plan are projected estimates only.

Liver Programs

A) Development Programs

1. AMT-021 for Acute Intermittent Porphyria

1.1.1 Disease Background

Acute Intermittent Porphyria, or AIP, is a rare liver metabolic disorder resulting from mutations in the PBGD gene. This gene encodes for the enzyme porphobilinogen deaminase (also known as hydroxymethylbilane synthase — HMBS), a liver protein necessary for the production of heme, a component of hemoglobin and other blood proteins. Insufficient activity of this protein leads to an accumulation of toxic metabolites (ALA and PBG), resulting in a wide variety of serious clinical problems, including acute, severe abdominal pain, muscular weakness and an array of neurologic manifestations, including psychiatric episodes, seizures and coma. In the majority of cases, attacks are triggered by precipitating factors such as hormonal fluctuations, infections, drugs and dietary changes. Long-term consequences may include irreversible nerve damage, liver cancer and kidney failure. Patients with AIP experience regular hospitalizations and extremely poor quality of life, and may in some cases require liver transplants. Acute attacks can be life-threatening. Current therapies only target the disease symptoms and do not prevent attacks or fully minimize or control their consequences.

A recent epidemiological study reported that, in Europe (excluding Sweden), the incidence of AIP is 0.13 per million population per year and based on that they estimated a prevalence of 5.9 per million population (Elder et al., 2012). In Sweden the incidence and prevalence of AIP are about four times higher than in the rest of Europe due to a founder effect originating in Lappland (Floderus et al., 2002). The frequency in the United States is estimated to be 1-5 cases per 100,000 population (www.emedicine.medscape.com/article/205220-overview#a0199).

1.1.2 Overview of AMT-021 Program

The goal of our AMT-021 program is to provide long-term normalization of the PBGD protein in order to prevent acute AIP attacks and their complications.

The program has been developed through a collaborative agreement with the Foundation for Applied Medical Research (FIMA), its Center for Applied Medical Research (CIMA) and its commercialization arm, DIGNA Biotech, of the University of Navarra (Pamplona, Spain). Part of the funding to support for the Phase I trial (including GLP safety & toxicology studies and the observational trial) was secured through the European Commission Framework Programme 7 award (€3.3 million, grant agreement 261506) made to the AIPGENE consortium (www.aipgene.org/), of which uniQure is a partner.

UniQure holds an exclusive license to the gene cassette being used in the Phase I clinical trial. Under our agreement with DIGNA Biotech and the other consortium members, **Licensee** have an exclusive right to all data related to the program.

1.1.3 Preclinical Development

· Product Profile

1.1.4 AMT-021 is designed to be delivered systemically through a peripheral vein in a single administration.

AMT-021 or rAAV5-hPBGD, is a recombinant adeno-associated vector of serotype 5, consisting of:

- Inverted terminal regions or ITRs of the adeno-associated serotype 2
- A human codon optimized porphobilinogen deaminase gene or hPBGDco as the therapeutic gene
- A liver specific promoter constituted by the albumin enhancer (Ealb) and the alfa-1-antitrypsin promoter (hAAT)

1.1.5

· Pre-clinical Proof of Concept

1.1.6 Pre-clinical proof of concept (PoC) studies have been performed using the AIP murine model developed by Lindberg et al. (1999). In these studies, long term therapeutic efficacy was achieved. More specifically, at 5x10<sup>13</sup> gc/kg, metabolic correction of the hepatic PBGD enzyme activity, normalization of the PBG and ALA precursor’s accumulation in urine and improvement of the motor coordination were observed. Additionally, a complete neurological study indicated the correction of neurotoxic porphyrin precursors was able to restore nerve conduction and the impaired peripheral neuropathy.

In non-human primates (NHP) treated with AMT-021 at a dose of 5x10<sup>13</sup> gc/kg endogenous PBGD enzymatic activity increased by a factor of two in male and between three and five times in female animals.

- Non-clinical safety & toxicology studies

1.1.7 The following table presents a summary of the AMT-021 non-clinical safety and toxicology studies that have been conducted to support the clinical development program.

Parameter to be assessed	Study performed	Results
[**]	[**]	[**]
[**]	[**]	[**]
[**]	[**]	[**]
[**]	[**]	[**]
[**]	[**]	[**]
[**]	[**]	[**]

1.1.8 Summary of AMT-021 Preclinical Development Program

Single intravenous administration of AMT-021 into wild type mice and Rhesus macaques results in:

- Efficient liver transduction resulting in dose dependent increase in viral RNA copy numbers and in turn producing increased PBGD activity

- No morbidity, no changes in body weight or food intake
- No changes in biochemistry, hematology, coagulation and urinalysis associated with AAV5-hPBGD
- Negative vector shedding [\*\*] days after viral administration in serum, saliva, nasal secretions, urine, faeces and semen
- Tissue biodistribution that is mainly limited to liver although some significant transduction was detected in spleen, lymph nodes, heart and adrenal glands
- Specific hepatic PBGD expression

1.1.9 Clinical Development Program

1.1.10 The key regulatory and clinical development best estimate milestones for AMT-021 include the following,

- |  |      |
|--|------|
| EMA Orphan Drug Designation (EU/3/09/632)            | [**] |
| FIMA/ CITA/ UTE/ DIGNA - AMT Collaborative Agreement | [**] |
| EU-FP7 AIPGene Consortium                            | [**] |
| Observational Study AEMPS approval                   | [**] |
| Observational Study start                            | [**] |
| Phase I Study AEMPS approval                         | [**] |
| Phase I Study: first patient treated                 | [**] |
| Phase I Study: last patient treated                  | [**] |

Expected milestones

- |                      |      |
|----------------------|------|
| Phase II/III start:  | [**] |
| MAA/ NDA submission: | [**] |
| Observational trial  |      |

1.1.11 A prospective non-interventional (pre-treatment) observational study started at the end of 2011 that aims to assess the evolution of disease-related clinical and laboratory parameters in time, as well as characterize aspects of disease management such as AIP-related hospitalization. This baseline assessment is intended to study possible relationships between biochemical parameters and clinical endpoints that will in turn be valuable in evaluating any signs of efficacy in the Phase I trial as

well as in subsequent trials. Eight patients are expected to be enrolled who after completion of this observational phase would then enter the interventional stage of the program, i.e., first-in-human clinical study (Phase I). The observational study is to last for at least six months for each participant.

1.1.12 To date all [\*\*] AIP-patients have been recruited into the observational study and all but one have completed a minimum of [\*\*] months pre-treatment assessments. The last patient completed the observational study in August 2013.

- Phase I trial

1.1.13 The Investigational Medicinal Product Dossier (IMPD) was submitted to the AEMPS (Spanish Agency for Medicines and Medical Devices) in June 2012 and was approved by the Agency in October 2012.

The Phase I study is a multicenter, open label, prospective, interventional, single dose, dose-escalation clinical trial to investigate the safety and tolerability of AAV5-hPBGDco (AMT-021) in patients with severe Acute Intermittent Prophyria (Eudra CT no. 2011-005590-23).

The primary objective is to assess the safety of systemic administration and determine the maximum tolerated doses (MTD). Secondary objectives include urinary levels of toxic metabolites (ALA and PBG), disease symptoms evaluation, quality of life evaluation and assessment of pharmacokinetics. Exploratory objectives include, neurological involvement, identification of novel biomarkers and pharmacokinetic modeling.

The Phase I study was initiated in December 2012 in the Department of Medicine (Liver Unit) at the University Clinic of the University of Navarra (Pamplona, Spain). There are [\*\*] patients per cohort and [\*\*] cohorts in the trial (each cohort receiving  $5 \times 10^{11}$ ,  $2 \times 10^{12}$ ,  $6 \times 10^{12}$  or  $1.8 \times 10^{13}$  gc/kg) and all patients will be followed-up for [\*\*] as part of the Phase I study.

All [\*\*] patients who completed the observational trial have also been treated as part of the Phase I study. In the 8 treated patients, no Serious Adverse Events, Treatment Emergent Adverse Events or Liver Events (Dose Limiting Toxicities - DLT's) related to the study medication have been observed to date.

- Future Clinical Development

It is envisaged that the Phase II/III will be a confirmatory trial where the study population and the outcomes to be assessed (efficacy endpoints — clinical and biochemical) will be based on those as for Phase I. **Licensee** also intend to carry out the study in both Europe and the USA.

#### 1.1.14 Summary of AMT-021 Clinical Development Program

- The first time an AAV5 gene therapy product has been tested in humans
- The first time an AAV gene therapy product has been tested in humans at such high dose, i.e.,  $1.8 \times 10^{13}$  gc/kg

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- No Serious Adverse Events, Treatment Emergent Adverse Events or Liver Events (DLT's) related to the study medication have been observed in the Phase I study to date
- The Phase I is expected to be completed in [\*\*] and Phase II/III is expected to start by the end of [\*\*]
- The Phase II/III program will run in parallel in Europe and US where MAA and NDA, respectively, are expected in [\*\*]

## 2. AMT-060 for Hemophilia B

### 1.1.1 Disease Background

Hemophilia B is a serious inherited orphan disease in males characterized by insufficient blood clotting. The condition can lead to repeated and sometimes life-threatening episodes of external and internal bleeding following accidental trauma or medical interventions. The episodes may cause long-term damage, for example to the joints, and may be fatal if they occur in the brain. The deficient blood clotting is caused by the lack of functional human Factor IX, or hFIX, a blood clotting factor, as a result of mutations in the gene responsible for encoding this essential protein. The presence of hFIX at greater than 1% of normal levels has a therapeutic effect in promoting clotting. The current standard treatment is prophylactic protein replacement therapy, in which frequent intravenous administrations of recombinant Factor IX (often 2-3 times per week) are required to stop or prevent bleeding. Protein replacement therapy is costly (\$150,000-200,000 per patient per year) and burdensome, and does not completely prevent bleeding.

The total Hemophilia B patient population in the European Union and the United States is estimated at approximately 25,000, according to the World Federation of Hemophilia 2010 Report on the Annual Global Survey. About 40% of individuals with the disease have a severe disorder, characterized by functional factor IX levels that are less than 1% of normal, whereas moderately severe Hemophiliacs (about 30% of the Hemophiliac population) have 1%-5% of normal and those with the mild phenotype (the remaining 30%) have between 5% and 40% of normal factor IX levels (www.orpha.net). Based on these estimates **Licensee** believes that approximately 70-85% of the worldwide patient population would be eligible for treatment with gene therapy. **Licensee** believes that the treatment would not be appropriate for those patients with very mild disease phenotype.

### 1.1.2 Overview of AMT-060 Program

The goal of our AMT-060 program is to restore blood clotting on a long-term basis through the introduction of the functional gene for hFIX into the patient's liver cells. **Licensee** is currently in the process of finalizing pivotal (GLP) safety and toxicology studies and preparing to conduct a Phase I trial.

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### 1.1.3 Preclinical Development

- Product Profile

#### 1.1.4 AMT-060 is designed to be delivered systemically through a peripheral vein in a single administration.

The use of recombinant adeno-associate vectors (rAAV) of serotype 5 (rAAV5) for targeted gene delivery to the liver was pioneered by St. Jude Children's Research Hospital (SICRH) where for pre-clinical experiments the hFIX expression cassette was packaged into AAV5 capsids in HEK-293T mammalian cells. HEK-293 produced AAV5-hFIX is not suitable for further development because as a production system it is not amenable to large-scale production. To allow up scaling, the expression cassette has now been transferred into uniQure's proprietary baculovirus expression vector system (BEVS) that can be adapted to a GMP setting. The resulting vector produced using the baculovirus expression system is termed AAV5-hFIXco or AMT-060. **Licensee** also holds a license from SICRH to the gene cassette used in the currently ongoing Phase I/II AAV 2/8-LP1-hFIXco trial.

AMT-060, rAAV5-hFIXco, is a recombinant adeno-associated vector of serotype 5, consisting of:

- Inverted terminal regions (or ITRs) of the adeno-associated serotype 2
- A human codon optimized FIX gene (or hFIXco) as the therapeutic gene
- The liver specific promoter, LP1, derived from the human apolipoprotein hepatic control region and the human alpha-1-antitrypsin (or hAAT) promoter
- *Virus serotype selection*

The hFIXco expression cassette and rAAV5 or AAV8 vectors have been extensively studied in mice and non-human primate. Both vectors have been shown to have similar tropism to (preference to transduce) the liver (Nathwani et al., 2007) and AAV5-hFIXco was shown to mediate expression of significant levels of human factor IX in non-human primates (NHP) during a monitoring period of more than 5 years (Nathwani et al., 2011). In this study none of the animals presented elevated liver enzymes levels or other signs of toxicity during the whole observation period. Liver examination by MRI scanning did not reveal any abnormalities in any of the animals.

These pre-clinical data suggest that i.v. administration of the AAV5-hFIXco vector is able to mediate a similar level of human factor IX as presented for AAV8-hFIXco, and such administration is not associated with safety concerns or immunogenicity against the human factor IX.

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#### · *Pre-clinical Proof of Concept*

Pre-clinical PoC studies have been carried out in wild type mice, non-human primates (NHP) and are currently being completed in transgenic Hemophilia B mice. In wild type mice (C57Bl/6) intravenous administration of AMT-060 mice resulted in dose-dependent levels of (human) factor IX levels in murine plasma as determined by ELISA. Human factor IX levels amounted up to 11% of those in normal human plasma 4 weeks after infusion of  $5 \times 10^{12}$  gc/kg, demonstrating that AAV5-hFIXco produced in the BEVS is biologically active.

In Rhesus monkeys dosed with AMT-060 ( $5 \times 10^{12}$  gc/kg) by intravenous infusion, human FIX levels peaked to 7%-16% of normal human levels one week after infusion, and stabilized to 5-10% of normal human levels 4 weeks after infusion until sacrifice (12 weeks after dosing). These kinetics are in accordance with those observed in previous studies (Nathwani et al., 2007; Jiang et al., 2006), indicating that i.v. administration of AAV5-hFIXco produced in BEVS results in a level of factor IX in plasma that is similar to that produced using AAV5-hFIXco produced in HEK293 cells. Post mortem, (RT)-QPCR demonstrated homogeneous vector DNA delivery and transgene expression in the liver. No signs of adverse reactions were observed. Infusion was associated with slight and transient effects in plasma chemistry shortly after dosing, such as a brief increase of liver enzyme activity levels, consistent with infusion of a viral protein. Necropsy revealed no significant macroscopic or microscopic abnormalities.

Preliminary data in Hemophilia B mice indicate that treatment with AMT-060 induces normalization of FIX levels as well as clotting time.

#### · *Non-clinical safety & toxicology studies*

The following table presents a summary of the AMT-060 non-clinical safety and toxicology studies that are being conducted to support the clinical development program.

Parameter to be assessed	Study performed	Status
***	***	***
***	***	***
***	***	***
***	***	***
***	***	***
***	***	***
***	***	***

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#### 1.1.5 Summary of AMT-060 Preclinical Development Program

- AAV5-hFIXco shows similar liver tropism to AAV8-hFIXco and results in significant and long lasting increase in FIX expression.
- Single intravenous administration of AMT-060 into wild type mice and Rhesus macaques results in significant and long lasting hFIX levels with no noticeable adverse events and no macroscopic or microscopic findings.
- GLP safety and toxicology studies are expected to be completed in \*\*\*.

#### 1.1.6 Clinical Development Program

#### 1.1.7 The key regulatory and clinical development milestones for AMT-060 include the following:

- EMA Orphan Drug Designation: [\*\*]
- FDA Orphan Drug Designation: [\*\*]
- EMA Scientific Advice: [\*\*]
- EMA Phase I Protocol Advice: [\*\*]
- GLP Safety & Tox Studies: [\*\*]

#### Expected milestones

- IMPD submission: [\*\*]
- Phase I start: [\*\*]
- Phase II/III start: [\*\*]
- MAA/ NDA submission: [\*\*]
- *Phase I trial*

The Phase I study will be a multicenter, open label, prospective, interventional, single dose, dose-escalation clinical trial to investigate the safety and tolerability of AAV5-hFIXco (AMT-060) in patients with severe Hemophilia B.

The primary objective is to assess the safety of systemic administration and determine the maximum tolerated doses (MTD). Secondary objectives include:

- To estimate the appropriate dose required to achieve stable expression of hFIX at or above 3% of normal
- To evaluate kinetics (dose-related duration and magnitude) of expression

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- To assess the immune response to hFIX transgene product
- To assess the immune response to the AAV5 capsid proteins
- To assess viral shedding in various body fluids (including semen)
- To assess the occurrence of FIX inhibitors
- To evaluate coagulation parameters
- To assess need for FIX concomitant treatment

[\*\*] male adults patients ( $\geq 18$  year old to  $\leq 35$  year old) with genetically confirmed Hemophilia B and phenotypically defined as having severe disease ( $\leq 1\%$  of normal plasma FIX levels) are expected to be enrolled. Initial patient follow-up will last for [\*\*] months as part of the Phase I trial.

- *Future Clinical Development*

1.1.8 It is envisaged that the Phase II/III will be a confirmatory trial where the study population and the outcomes (efficacy endpoints — clinical and biochemical) will be based on those for the Phase I. **Licensee** will also consider expanding the patient population to moderately severe patients and intend to carry out the study in both Europe and USA.

#### 1.1.9 Summary of AMT-060 Clinical Development Program

- **The IMPD is planned to be submitted in [\*\*]**
- **Phase I is planned in patients with severe Hemophilia B and is expected to start in [\*\*]**
- **[\*\*]**
- **The Phase II/III program will run in parallel in Europe and USA where MAA and NDA, respectively, are expected in [\*\*]**

The Hemophilia B program has been partnered with Chiesi. The co-development agreement has been shared with NIH.

### B) Active Research Projects

#### 1. Hemophilia A

**Disease Background:** Hemophilia A (HA) is a genetic, X-linked, recessive disorder caused by production of dysfunctional or by production of insufficient amount of factor VIII (FVIII) protein, a key protein involved in the blood coagulation cascade. Hemophilia A patients suffer from spontaneous bleeding in the large joints and soft tissue, and are at risk for intracranial hemorrhage. Recurrent episodes of joint bleeding can lead to crippling arthropathy, particularly in severely affected patients. HA comprises the majority of hemophilia patients (80%), with incidence of  $\sim 1:10,000$  to  $1:50,000$  males affecting 400,000 people worldwide.

Numerous mutations in the FVIII gene have been described giving rise to different disease phenotypes. Similarly to



Hemophilia B (HB), individuals with less than 1% active factor are classified as having severe hemophilia, those with 1—5% active factor have moderate hemophilia, and those with mild hemophilia have between 5—40% of normal levels of active clotting factor.

**Clinical need:** HA seems an excellent candidate for gene therapy (GT) as it is a well characterized monogenic disorder. The product of the FVIII gene is a plasma protein which is normally secreted by hepatocytes and endothelial cells but can also be expressed in other cell types, e.g., adipocytes, myocytes or fibroblasts. Furthermore, only modest increase >1% can markedly reduce spontaneous bleedings. The effects of gene therapy can be readily monitored by changes in phenotype and by obtaining peripheral blood to measure FVIII antigen levels and clotting factor activity. Currently, treatment for HA consists of infusion of either plasma-derived or rFVIII protein for bleeding episodes. Although, prophylactic infusion of FVIII concentrates is generally effective in alleviating bleeding episodes and subsequent joint disease, the short half-life of FVIII (~12 hours) and the high cost of purified FVIII products make life-long prophylactic treatment demanding for patients and costly.

Feasibility

**Gene:** The gene of factor VIII is located on the long arm of the X chromosome. It spans over 180 kb, and as such is one of the largest genes known. It comprises of 26 exons, which encode a polypeptide chain of 2351 amino acids including a signal peptide of 19 and a mature protein of 2332 amino acids. It is a secreted protein. Its primary structure, deduced from the cloned factor VIII cDNA, includes discrete domain structure: A1-a1-A2-a2-B-a3-A3-C1-C26-8. The B domain is unique in that it exhibits no significant homology with any other known protein and can be deleted with the resulting recombinant protein displaying essentially normal survival in circulation and able to correct the bleeding tendency in HA patients.

[\*\*]  
A proof of concept study has been initiated involving a number of FVIII construct and including full FVIII codon optimized gene. The study aims to characterize the viral DNA, formation of episomes upon delivery of the expression cassette to the nucleus, resulting mRNA and FVIII protein. The potency of the vector is currently being investigated in a number of animal models.

It is our aim to develop this product to clinical stage Phase I by the [\*\*]. Duration of clinical development and further timelines have not been defined.

Development overview to IMPD:

[\*\*]



Completion of vector optimization work will provide the first milestone (Go/No Go) for the project.

**Safety Assessment:** The disease and gene therapy approach are similar (or equivalent) to Hemophilia B where no major safety concerns have been described.

2. Cirrhosis

**Disease Background:** Liver cirrhosis is a consequence of chronic liver disease characterized by replacement of liver tissue by fibrosis, scar tissue and regenerative nodules (resulting from regeneration of damaged tissue), leading to loss of liver function. The four leading causes of cirrhosis and primary liver cancer in Europe include harmful alcohol consumption, viral hepatitis B, viral hepatitis C and metabolic syndromes related to overweight and obesity. The European Association for the Study of the Liver in its 2013 report reported that approximately 29 million people in the European Union suffer from a chronic liver condition and that the incidence and prevalence of two conditions, cirrhosis and primary liver cancer, are key to understanding the burden of liver disease. Both conditions represent the end-stage of liver pathology and thus are indicative of the associated mortality.

The hypothesis behind this project is that liver cirrhosis is a state of IGF-I insufficiency and low expression of IGF-I locally in the liver will revert and/ or prevent further exacerbation of cirrhosis. A confidentiality agreement concerning this project was signed between DIGNA/ CIMA and uniQure in October 2012.

[\*\*]



Clinical evidence to support disease linkage includes the following:

- In patients suffering from liver cirrhosis circulating IGF-I levels (or IGF-BP3) correlate with disease severity scores; Child-Pugh and MELD (Kratzsch et al., 2005; Khoshnood et al., 2013).
- A short course (for 4 months) of IGF-I recombinant therapy treatment increased the levels of albumin and tended to improve energy metabolism (surrogates for liver function) & the levels of serum albumin positively correlated with IGF-I/IGF-I BP3 ratio (Conchillo et al., 2005).

**Clinical need:** Transplantation is the only curative option for the disease and contraindications to transplantation include, a) co-morbidities (e.g., TB), b) over 65 years of age, c) coronary artery disease and d) tumours in previous 5 years.

The initial target population for IGF-I gene therapy for liver cirrhosis could/ would be those cirrhotic patients with IGF-I insufficiency (i.e., 50% of all cirrhotic patients), possibly patients with Child-Pugh A and/ or B score and with IGF-I levels below normal values. An ODD application for this specific population may be considered. The table below indicates the Child-Pugh scoring scheme for liver disease prognosis.

Points	Class	One year survival	Two year survival
5-6	A	100%	85%
7-9	B	81%	57%

**Feasibility:**

**Gene:** The IGF1 gene is located on chromosome 12 and spans 7.3 kb encoding a 70 amino acid residue protein. It contains 6 exons, 4 of which are alternatively spliced depending on tissue type and hormonal environment. The IGF1 coding region is flanked by sequences encoding an amino-terminal peptide of at least 25 residues and a carboxyl-terminal peptide of 35 amino acids which indicates that IGF1 is synthesized as a precursor protein that undergoes proteolytic processing at both ends before being secreted.

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**Animal models:** A rat model is available with CIMA and has been used for proof of concept studies. A number of other small animal models have been described (Liu et al., 2013).

**Biomarkers:** Circulating IGF-I (and other related proteins) can be monitored using commercially available methodology. However the relevance of this to liver (local) levels of IGF-I and whether GT can deliver sufficient amounts of IGF-I that that can be readily detectable in the circulation need to be established.

Liver function and signs of cirrhosis can be monitored following well established standard procedures (e.g., liver enzymes, markers of fibrosis etc.).

The PoC obtained at CIMA will have to be repeated with uniQure's AAV5-IGF1 vector. **Licensee** is at the initial stages of research aiming to initiate a Phase I clinical trial by the [\*\*].

**Development overview to IMPD:**

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The GLP safety and toxicology studies will provide the first milestone (Go/No Go) for the project.

**Safety Assessment:** Safety studies in rat disease models (8 months) and wild type rats (8 weeks) showed no signs of toxicity due to treatment with SV40-IGF-I (Sobrevals et al., 2010).

Potential toxicity concerns include tumor formation and interference with insulin/ glucose metabolism albeit both issues are unlikely as the aim of this approach would be to upregulate levels of IGF-I where they are already below normal rather than to achieve supra-physiological levels. In addition, gene therapy vectors are likely to induce lower level of localized expression without substantial increase in serum IGF-I levels. Regarding potential for tumorigenesis, IGF-I therapy is thought to favor hepatocellular differentiation, i.e., opposes carcinogenesis, and studies have shown that sharp decrease in IGF-I in cirrhotic liver may contribute to hepatocellular carcinoma (HCC). In addition it is believed that it is IGF-II that is the key player in HCC. Furthermore, patients with existing tumor nodules in their liver could/ should be excluded from trials.

[NOTE: Hepatocellular carcinoma occurs at a rate of 1% to 4% per year after cirrhosis is established and cirrhosis underlies HCC in approximately 80%-90% of cases worldwide (Giovanna Fattovich et al., 2004), i.e., the vast majority of cirrhotic patients do not develop HCC or at least they do not live long enough to develop it]

**3. Hyperoxaluria**

**Disease Background:** Primary hyperoxaluria type I (PH1) is a rare, autosomal recessive inherited metabolic disorder characterized by a deficiency of the hepatic enzyme alanine-glyoxylate aminotransferase (AGXT), which produces a marked increase in endogenous oxalate synthesis by the liver. Oxalate is a metabolic end product in humans and excess oxalate provokes hyperoxaluria, causing progressive urolithiasis, nephrocalcinosis and chronic renal failure, ultimately leading to end-stage renal failure (ESRF) and death if untreated.

It is the most common and severe variant among a spectrum of metabolic disorders resulting in hyperoxaluria. The disease has an estimated prevalence ranging from 1 to 3 per 1 million individuals and an estimated incidence of 1-9:100,000 live births per year in Europe. However, higher rates are reported in historically isolated populations, like the Canary Islands. PH1 accounts for <1% of pediatric ESRF in developed countries.

A pre-clinical proof of concept study has already been conducted in collaboration with Eduardo Salido (University Hospital of Canary Islands) using AGXT knockout mice demonstrating that in the GT treated animals oxalurea reduced to normal levels with restoration of liver enzyme levels in the absence of any hepatotoxicity or immune reactions.

**Clinical need:** Currently, most of the therapeutic options are diet-mediated to reduce the amount of glyoxylate intake and maximize the intake of vitamin B6. The most effective treatment for PH1 is pre-emptive liver transplantation, alone or liver combined with kidney transplantation in ESRF. There is therefore a clear need for

alternative or new treatments options.

**Feasibility:**

**Gene:** the AGXT gene maps onto chromosome 2q36-q37, has a 10 kb coding sequence and contains 11 exons generating a 392-residue protein.

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**Animal models:** Small animal models already exist and have been used for pre-clinical proof of concept studies.

**Biomarkers:** Measurements of oxalate are part of routine clinical practice for the disease setting and monitoring of kidney changes can also be done using standard techniques.

After a phase of further vector optimization it is our aim to develop this product for a first Phase I clinical study by [\*\*]. Further development timelines have not been defined.

**Development overview to IMPD:**

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The GLP safety and toxicology studies will provide the first milestone (Go/No Go) for the project.

**Safety Assessment:** At this stage is not possible to make any inferences in relation to potential safety concerns.

**C) Exploratory Research Projects**

The projects listed under this category in Table 1 above are not in active research yet, but are likely targets for our platform technology and are being assessed on feasibility before starting active bench work.

**Central Nervous System Programs**

**A) Development Programs**

1. AMT-110 for Sanfilippo B

1.1.1 *Disease Background*

Sanfilippo syndrome, or Mucopolysaccharidosis type III (MPSIII), is a rare lysosomal storage disorder (LSD) that occurs when enzymes needed to break down the heparan sulfate sugar chain are missing or are defective. Sanfilippo B is one of the four types of MPSIII that results in serious brain degeneration in children, and is generally lethal. The deficient enzyme responsible for the disease is alpha-N-acetylglucosaminidase (NaGlu). The clinical manifestations are mainly neurological, with early symptoms observed during the first five years of age, leading to a progressive deterioration of cognitive abilities. Affected children require specific care after age seven and progressively develop profound mental retardation with reduced somatic manifestations. Death frequently occurs at the median age of 15. No treatment is currently available.

Birth prevalences of 0.28—4.1 per 100, 000 have been reported (Valstar et al., 2008). More recently, He´ron et al. (2010) estimated the mean annual incidence for Sanfilippo B in France at 0.15 per 100,000 births.

1.1.2 *Overview of AMT-110*

The goal of our AMT-110 program is to provide a gene therapy for Sanfilippo B syndrome through the introduction of a functional NaGlu gene into the patients’ brain cells.

This project is being pursued together with the Pasteur Institute (Paris) whereby uniQure is responsible for developing the manufacturing process and producing clinical grade material and the Pasteur Institute for conducting the clinical trials.

1.1.3 *Preclinical Development*

· *Product Profile*

AMT-110 is designed to be delivered via intracranial administration.

AMT-110 or rAAV5-hNaGlu, is a recombinant adeno-associated vector of serotype 5, consisting of:

- Inverted terminal regions or ITRs of the adeno-associated serotype 2
- A human α-N-acetylglucosaminidase, or hNaGlu, gene the therapeutic gene
- The mouse phosphoglycerate kinase-1 promoter (muPGK)

· *Pre-clinical Proof of Concept*

Preclinical PoC studies were conducted in mouse and dog disease models at the Pasteur Institute. These studies showed that mice with MSPIIB a single AAV5-NaGlu intracranial injection resulted in reversion of storage lesions throughout the brain and prevented loss of Purkinje cells. Furthermore, it improved animal behavior and corrected pathological featured of the disease including, neuro-inflammation, axonal transport, synaptic vesicle content and the autophagy defect.

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· *Non-clinical safety & toxicology studies*

The following table presents a summary of the AMT-10 non-clinical safety and toxicology studies that have been conducted to support the clinical development program.

Parameter to be assessed	Study performed	Results
***	***	***
***	***	***
***	***	***

#### 1.1.4 Summary of AMT-110 Preclinical Development Program

- In animal models of Sanfilippo B, treatment with AAV5-hNaGlu ameliorated pathophysiological signs and symptoms of the disease.*

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- AMT-110 administered into the striatum of non-immunosuppressed rats and immunosuppressed rats and dogs produced long lasting presence of vector DNA in the brain and caused no mortality and no signs of toxicity.*

1.1.5

1.1.6 Clinical Development Program

1.1.7 *The key regulatory and clinical development milestones for AMT-110 include the following,*

- 1<sup>st</sup> Scientific Advice with French Regulatory Authorities [\*\*]
- 2<sup>nd</sup> Scientific Advice with French Regulatory Authorities [\*\*]
- IMPD Submission [\*\*]
- IMPD Approval [\*\*]
- Phase I start [\*\*]

Expected Milestones

- Phase II/III start [\*\*]
- Registration [\*\*]

The Phase I/II study is a single center, open label, prospective, interventional, single dose of AAV5-hFIXco (AMT-060) trial in children with Sanfilippo type B syndrome. [\*\*].

The primary objective of the study is to evaluate the clinical, radiological and biological safety of the treatment. The secondary objective is to collect samples and data to define exploratory tests that could become evaluation criteria for further clinical efficacy studies (Brain MRI; neurological tests and biological markers).

The study will be conducted at the Bicêtre Hospital which is part of the University Hospitals of South Paris and is expected to enroll a total of [\*\*] children during an [\*\*] months inclusion period. The duration of follow-up for each patient is [\*\*]. The first patient was dosed in October 2013.

- Future Clinical Development

**Licensee** plans to complete the Phase I and start a Phase II/III trial in multiple sites worldwide. Following initiation of this trial one of the options on how to proceed would be applying for approval for compassionate use to treat on a named patient basis. This can be well justified based on the size of the indication and lethality of the condition.

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#### 1.1.8 Summary of AMT-110 Clinical Development Program

- The IMPD was submitted in [\*\*]**
- Phase I was started in [\*\*]**

### B) Active Research Projects

#### 1. Huntington's Disease

**Disease background:** Huntington's Disease (HD) is a neurodegenerative genetic disorder that affects motor control and leads to cognitive decline and dementia. It typically becomes noticeable in middle age, but can begin at any age from infancy to old age. HD has a prevalence of around 1 affected individual in 100,000.

The mutated form of the protein huntingtin causes cellular dysfunction and death in a number of CNS sites but is most noticeable in the striatum and cortex. The mutation is caused by CAG repeats in the DNA of patients. The earliest features of HD are involuntary movements and irritability and a loss of executive function. This progresses over time and in the more advanced stages, the patient is demented and bed-bound. The disease is currently incurable with patients dying about 20-25 years after it begins.

**Clinical need:** The clinical need for these patients is high as there is no cure for the disease.

## Feasibility

As the CAG repeats in the Huntingtin gene are the cause of the disease, downregulation of the expression of the CAG repeats is an option. Also rescuing the neurons from degeneration using GDNF is an option. Both options are currently under investigation. Replacing the gene is not an option as this is far too large to fit into an AAV vector.

Several transgenic mice models exist. Severity and time of onset are based on the number of CAG repeats in the model. Mostly used are the R6/1 and R6/2 transgenic models.

**Preliminary work:** Proof of concept using GDNF has been established in one laboratory. **Licensee** is currently trying to establish this with our own vector in the laboratory of Roger Barker.

Proof of concept with siRNA has been established in mice models and **Licensee** is in the process of implementing this into our studies.

### Development overview to IMPD:

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The proof of concept studies (*in vivo/ in vitro* work) will provide the first milestone (Go/No Go) for the project.

With regards to the siRNA approach to HD, vector generation & optimization will require an additional 9 months prior to any other activity. Then a similar development path to what is shown above will need to be

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followed.

It is **Licensee's** aim upon a successful PoC to develop this product further to a Phase I clinical investigation which should start [\*\*].

**Collaborators:** **Licensee** is working together with Roger Barker (Cambridge University) on the use of GDNF to rescue neurons in Huntington models, based on a EUREKA grant. **Licensee** is also working together with Nicole Deglon (Lausanne University), Anna Skorupska (Lublin University) and Sebastian Kuegler (Gottingen University) in a Eurostars grant setting. Competition comes from siRNA companies.

**Safety concerns:** Potential safety concerns could be the complete downregulation of the Huntingtin gene, even though not fully supported by the Eurostars team. The use of GDNF could lead to side effects, such as weight loss.

**IP:** For GDNF, **Licensee** has a license from Amgen. For the siRNA work **Licensee** has a non-exclusive license from Benitec.

## 2. Multiple System Atrophy

**Disease Background:** Multiple System Atrophy (MSA) is a sporadic neurodegenerative disease that is characterized by the presence of glial inclusion bodies, which stain positive for a synuclein. The clinical picture is that of parkinsonism, autonomic failure, cerebellar ataxia and pyramidal signs in differing combinations. Approximately 80% of patients present with predominantly parkinsonian features (MSA-P) manifesting in rapidly deteriorating akinesia, rigidity, postural instability and high pitched dysarthria. Most such patients do not exhibit the classic resting tremor of Parkinson's disease and virtually all develop frank dysautonomia in the course of the illness. The cause of the disease is not known.

**Clinical need:** Although a minority of patients may achieve modest benefit from dopaminergic therapy, there is no satisfactory treatment for the parkinsonian disabilities of MSA-P. Additionally, deep brain stimulation of the subthalamic nucleus has been of little or no value. Within 5 years of disease onset patients die so the clinical need is high for these patients.

### Feasibility:

MSA is not a single monogenic disease, but may be treated with a single neuroprotective protein. In this case, this could be GDNF. Some transgenic animal models exist, all overexpressing the alpha-synuclein protein. The rationale to use GDNF (besides its general neuroprotective effect on neurons) is that both in patients and the transgenic mouse model, GDNF expression is downregulated. Introduction of an elevated level of GDNF may serve as the treatment. Read out parameters for the disease progression are all related to those of Parkinson's Disease. PoC has not yet been established, but is under investigation in the mouse model.

### Development overview to IMPD: a

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The proof of concept studies (*in vivo/ in vitro* work) will provide the first milestone (Go/No Go) for the project.

It is our aim upon a successful PoC to develop this product further to a Phase I clinical investigation which should start [\*\*].

**Collaborators:** **Licensee** is working together with Erwan Bezard (University of Bordeaux) and Olivier Rascol (University of Toulouse) who are together running the French reference center for MSA.

**Safety Assessment:** The use of GDNF could lead to side effects, such as weight loss. The exact mechanism through

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which the treatments would have its effect is not clear yet.

### 3. Hearing loss

**Disease background:** Hearing loss is a serious clinical problem. Underlying mechanisms for the loss of neurons in the cochlea can vary from ischemia, mechanical stress to toxic insults. The actual numbers of patients is not easy to define, but it could be rather large. When age-related hearing loss is also taken into account, this is no longer an orphan indication.

**Clinical need:** Patients with hearing loss could be helped with cochlear implants. However, progressive neurodegeneration is not stopped by that. There is high clinical need as there is no cure for the disease.

#### Feasibility:

Neuron function and survival is dependent on a delicate balance of neurotrophins. Following trauma or toxic insult to neurons, they may slowly die. To reverse this state of degeneration, it could be beneficial to supply the neurons with a neurotrophin such as GDNF. This neurotrophin has been shown to be able to rescue neurons from degeneration in several models, including those of the substantia nigra and for instance motoneurons in the spinal cord after trauma.

Animal models are available and include for instance use of Kanamycin in cats, mice or guinea pigs. Also chemotherapeutic agents from the class of statins are used.

**Preclinical work:** Proof of concept using recombinant brain-derived neurotrophic factor (BDNF) and/or GDNF has been established. **Licensee** is currently trying to establish this with our own vector in the laboratory of Patricia Leake.

Cochlea of mice can be transduced to express a recombinant transgene.

#### Development overview to IMPD:

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The proof of concept studies (*in vivo/ in vitro* work) will provide the first milestone (Go/No Go) for the project.

This new project has just been initiated upon a successful PoC it is our aim to develop this product further to a Phase I clinical trial, which should start by the [\*\*].

**Collaborators:** **Licensee** is working together with Patricia Leake (University College of San Francisco) on the use of GDNF to rescue neurons in mouse and cat models. She is the investigator who developed the cochlear implant. This could also be included in the experimental plan.

**Safety concerns:** The use of GDNF could lead to side effects. Weight loss is not expected, but as the GDNF also has a neurotrophic effect, nerve fibers could sprout in an aberrant way possibly leading to incorrect connections.

**IP:** For GDNF, **Licensee** has a license from Amgen; the program as a whole is under investigation.

### C) Exploratory Research Projects

The projects listed under this category in Table 1 above are not in active research yet, but are likely targets for our platform technology and are being assessed on feasibility before starting active bench work.

Confidential Materials omitted and filed separately with the Securities and Exchange Commission. Double asterisks denote omissions.

### License Agreement- Non-Exclusive

(*expresSF*<sup>+</sup>® cells)

This License Agreement (the “Agreement”) is entered into and made effective the 22nd day of March, 2007, (the “Effective Date”) between PROTEIN SCIENCES CORPORATION, whose principal place of business is at 1000 Research Parkway, Meriden, CT 06512 USA (hereinafter referred to as “LICENSOR”) and AMSTERDAM MOLECULAR THERAPEUTICS whose principal place of business is at Meibergdreef 61, P.O. Box 22506, 1100 DA Amsterdam, The Netherlands, (hereinafter referred to as “LICENSEE”).

#### WITNESSETH

WHEREAS, LICENSOR is the assignee of U.S. Patent No. [\*\*];

WHEREAS, LICENSEE desires to acquire a non-exclusive worldwide license under the Licensed Patent and to use the Technology for Product research, development and commercialization purposes; and

WHEREAS, LICENSOR warrants that it possesses the right to license the Licensed Patent (as herein defined);

NOW THEREFOR, for these and other valuable considerations, the receipt of which is hereby acknowledged, the parties agree as follows:

#### 1. DEFINITIONS:

1.1 “Affiliate” shall mean any corporation or other business entity controlled by, controlling or under common control with LICENSOR or LICENSEE. For this purpose, “control” shall mean direct or indirect beneficial ownership of at least fifty percent (50%) of the voting stock of, or at least a fifty percent (50%) interest in the income of, such corporation or other business entity, or such other relationship as, in fact, constitutes actual control.

1.2 “Product(s)” shall mean any recombinant Adeno-associated Virus (rAAV) vector product (or part thereof) that is developed as a direct result of LICENSEE’s use of the Technology and which product (or part thereof) is listed in Appendix I to this Agreement, it being understood that Appendix I may be amended during the term of this Agreement to incorporate new Product(s) developed by LICENSEE.

1.3 “Technology” shall mean the cells marketed by LICENSOR as *expresSF*<sup>+</sup>® cells, or the cells deposited by LICENSOR at the ATCC as CRL-12579, and any progeny or derivatives thereof.

1.4 “Territory” shall mean the world.

1.5 “Licensed Patent” shall mean claims 1-8 of U.S. Patent No. [\*\*] and any counterparts thereto in the Territory.

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1.6 “Intellectual Property Rights” shall mean the Licensed Patent, the Technology and the know how associated therewith.

#### 2. GRANT:

2.1 In consideration for payment of fees, LICENSOR hereby (i) grants to LICENSEE a non-exclusive license under the Licensed Patent and the Technology and know how involving the Technology in the Territory, without the right to sublicense, and (ii) transfers cells, both for the following uses: to research and have researched, to develop and have developed, to manufacture and have manufactured, to import and have imported, to market and have marketed and to offer for sale and sell Products anywhere in the Territory (“Permitted Acts”). By this Agreement, LICENSOR is not granting — either expressly or by implication — any licenses under any other patents or technology owned or licensed by LICENSOR, including U.S. Patents Nos. [\*\*].

2.2 LICENSEE agrees that the Technology is covered by the Licensed Patent and that if LICENSEE challenges the validity of the Licensed Patent or uses any allegation of invalidity — either by LICENSEE or a third party — as a basis for non-payment of fees hereunder, then LICENSOR shall have the right to terminate this Agreement pursuant to Section 12.1.

2.3 LICENSEE agrees that if either party makes commercially useful derivatives, developments or improvements to, from, of or utilizing Intellectual Property Rights, such derivatives, developments and improvements and all intellectual property rights therein, including patent rights, shall belong to LICENSOR. LICENSEE shall promptly notify LICENSOR of such derivatives, developments and improvements, and LICENSOR in its sole discretion shall determine whether or not to pursue intellectual property protection therefor. At LICENSOR’s request, LICENSEE and its employees and agents shall assign to LICENSOR the intellectual property rights in, to and under the derivatives, developments and improvements to, from, of or utilizing the Intellectual Property Rights, without any additional consideration from LICENSOR, and execute all necessary documents so that LICENSOR may pursue intellectual property protection for the derivatives, developments and improvements. As compensation for assigning the intellectual property rights in, to and under the derivatives, developments and improvements, LICENSOR hereby grants to LICENSEE a non-exclusive royalty-free license under those intellectual property rights, under the terms of this Agreement; said intellectual property rights shall thus be included in the term “Licensed Patent” as used herein. For clarity, all conceptions, inventions, discoveries, data, information, or any results whatsoever and wheresoever generated by or upon behalf of LICENSEE in carrying out the Permitted Acts that relate solely and exclusively to only the Product and all intellectual property (including secret know how) or property solely and exclusively covering or relating thereto shall be solely owned by the LICENSEE, provided that, effective from and after termination of this Agreement, LICENSOR is hereby granted a royalty-free, perpetual, irrevocable, sublicensable non-exclusive license therein in the Territory.

2.4 LICENSOR agrees to provide regulatory and technical support in the manufacturing of the LICENSEE’s master (working) cell bank (MCB/WCB) and the LICENSEE’s master (working) virus bank (MVB/WVB) using the Technology. The regulatory support will include a documentation package regarding history and characterization of the Technology for cGMP manufacturing, including the signed certificate of analysis for WCB #040704 or any other MCB

owned by PSC of which a vial will be send to AMT. The fees payable by the LICENSEE hereunder shall include ten hours of technical and FDA or EMEA/CHMP regulatory assistance from the LICENSOR. Any hours exceeding this will be billed at \$[\*\*] USD/hour.

2.5 LICENSEE agrees to provide LICENSOR with EMEA/CHMP filing reference numbers so that LICENSOR can refer to such filings solely in relation to the LICENSOR's corresponding regulatory filings covering the Technology.

### **3. TERM:**

This Agreement and the license granted by this Agreement shall be non-exclusive for a term commencing as of the Effective Date of this Agreement and continuing thenceforth subject to payment of the fees set out in Section 8, unless terminated pursuant to Section 12.

### **4. UNITED STATES LAWS:**

4.1 It is understood that LICENSOR is subject to United States laws and regulations controlling the export of technical data, computer software, laboratory prototypes and other commodities (including the Arms Export Control Act, as amended and the Export Administration Act of 1979), and that its obligations hereunder are contingent on compliance with applicable United States export laws and regulations. The transfer of certain technical data and commodities may require a license from the cognizant agency of the United States Government and/or written assurances by LICENSEE that LICENSEE shall not export data or commodities to certain foreign countries without prior approval of such agency. LICENSOR neither represents that a license shall not be required nor that, if required, it shall be issued.

### **5. USE OF TRADEMARK:**

5.1 LICENSOR shall have no responsibility with respect to LICENSEE'S own trademarks and tradename, and LICENSEE in respect to the use thereof will defend, indemnify and hold harmless LICENSOR against any and all third party claims. LICENSEE agrees that LICENSOR may include LICENSEE's name in any listings by LICENSOR of LICENSOR's licensees. LICENSEE shall not use LICENSOR's names or marks without the express written permission of LICENSOR.

### **6. INDEMNIFICATION:**

6.1 LICENSEE agrees to release, indemnify and hold harmless the LICENSOR, its directors, officers and employees against any and all losses, expenses, claims, actions, lawsuits and judgments thereon (including attorney's fees through the appellate levels) which may be brought against LICENSOR, its directors, officers and employees as a result of or arising out of use, production, manufacture, sale, lease, consumption or advertisement by, or on behalf of, LICENSEE of any Product or Technology licensed under this Agreement.

### **7. WARRANTY:**

7.1 LICENSOR MAKES NO WARRANTIES, EXPRESS OR IMPLIED, AND HEREBY DISCLAIMS ALL SUCH WARRANTIES, AS TO ANY MATTER WHATSOEVER,

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INCLUDING, WITHOUT LIMITATION, THE CONDITION, INCLUDING PURITY, OF ANY INVENTION(S), TECHNOLOGY OR PRODUCT, WHETHER TANGIBLE OR INTANGIBLE, LICENSED UNDER THIS AGREEMENT; OR OF MERCHANTABILITY, OR FITNESS FOR A PARTICULAR PURPOSE OF THE INVENTION, TECHNOLOGY OR PRODUCT; OR OWNERSHIP; OR THAT THE USE OF THE LICENSED PATENT, TECHNOLOGY OR PRODUCT WILL NOT INFRINGE ANY PATENT, COPYRIGHTS, TRADEMARKS, OR OTHER RIGHTS. LICENSOR SHALL NOT BE LIABLE FOR ANY DIRECT, CONSEQUENTIAL, OR OTHER DAMAGES SUFFERED BY ANY LICENSEE OR ANY THIRD PARTIES RESULTING FROM THE USE, PRODUCTION, MANUFACTURE, SALE, LEASE, CONSUMPTION, OR ADVERTISEMENT OF THE TECHNOLOGY OR PRODUCT.

7.2 LICENSOR represents and warrants that is has good title to the Technology and Licensed Patent and has the right to license the Licensed Patent as provided herein. LICENSEE agrees to indemnify, hold harmless, and defend LICENSOR from and against any and all claims, loss, damages, or expenses arising out of any third party claims of infringement resulting from the use by LICENSEE of the Technology or out of the use, sale or other disposition of Products.

### **8. FEES AND CELLS:**

8.1 In consideration of the license herein granted, LICENSEE shall pay fees to LICENSOR as follows:

(a) License Issue Fee of \$50,000 that shall be deemed earned and due immediately upon the execution of this Agreement, at which time the LICENSEE shall be provided with a supply of [\*\*].

(b) A license fee of \$50,000 for every additional product added to Appendix I upon the addition of such Product to Appendix I; and

(c) License Maintenance Fees of \$[\*\*] on each one-year anniversary thereafter, upon receipt of which by LICENSOR, the LICENSEE be provided with a supply of up to [\*\*] if the LICENSEE so requests.

8.2 All payments shall be made hereunder in U.S. dollars.

### **9. MARKING AND STANDARDS:**

9.1 LICENSEE agrees to maintain satisfactory standards in respect to the nature of the Product manufactured and/or sold by LICENSEE. LICENSEE agrees that all Products manufactured and/or sold by it shall be of a quality that is appropriate to products of the type here involved. LICENSEE shall use appropriate patent marking on Products with respect to the Licensed Patent.

### **10. ASSIGNMENT:**



10.1 This Agreement is not assignable by LICENSEE or by operation of law without the prior written consent of LICENSOR at its sole discretion.

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10.2 This Agreement shall extend to and be binding upon the successors and legal representatives and permitted assigns of LICENSEE. This Agreement shall extend to and be binding upon the successors and legal representatives and assigns of LICENSOR.

## **11. NOTICE:**

Any notice, payment, or other correspondence (hereinafter collectively referred to as “correspondence”) required or permitted to be given hereunder shall be mailed by certified mail or delivered by hand to the party to whom such correspondence is required or permitted to be given hereunder. If mailed, any such notice shall be deemed to have been given when mailed as evidenced by the postmark at point of mailing. If delivered by hand, any such correspondence shall be deemed to have been given when received by the party to whom such correspondence is given, as evidenced by written and dated receipt of the receiving party.

All correspondence to LICENSEE shall be addressed as follows:

Sander J.H. van Deventer  
Chief Scientific Officer  
Amsterdam Molecular Therapeutics  
Meibergdreef 61  
1105 BA Amsterdam  
The Netherlands

All correspondence to LICENSOR shall be addressed as follows:

Manon M.J. Cox  
Chief Operating Officer  
Protein Sciences Corporation  
1000 research Parkway  
Meriden, CT 06512 USA

Either party may change the address to which correspondence to it is to be addressed by notification as provided herein.

## **12. TERMINATION:**

12.1 LICENSOR and LICENSEE shall have the right to terminate this Agreement if the other party commits a material breach of an obligation under this Agreement and continues in default for more than [\*\*] months after receiving written notice of such default. Such termination shall be effective upon further written notice to the breaching party after failure by the breaching party to cure such default.

12.2 LICENSEE agrees that breach of terms of this Agreement would immediately and irreparably damage LICENSOR in a way not capable of being fully compensated by monetary damages and accordingly, the LICENSOR is entitled to injunctive relief in addition to such other relief to which it may be entitled at law or in equity.

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12.3 The license and rights granted in this Agreement have been granted on the basis of the special capability of LICENSEE to perform research and development work leading to the manufacture and marketing of the Product. Accordingly, LICENSEE covenants and agrees that in the event any proceedings under the Dutch Bankruptcy Act or any amendment thereto, be commenced by or against LICENSEE, and, if against LICENSEE, said proceedings shall not be dismissed with prejudice before either an adjudication in bankruptcy or the confirmation of a composition, arrangement, or plan of reorganization, or in the event LICENSEE shall be adjudged insolvent or make an assignment for the benefit of its creditors, or if a writ of attachment or execution be levied upon the license hereby created and not be released or satisfied within ten (10) days thereafter, or if a receiver be appointed in any proceeding or action to which LICENSEE is a party with authority to exercise any of the rights or privileges granted hereunder and such receiver be so discharged within a period of forty-five (45) days after his appointment, any such event shall be deemed to constitute a breach of this Agreement by LICENSEE and, LICENSOR, at the election of LICENSOR, but not otherwise, ipso facto, and without notice or other action by LICENSOR, shall terminate this Agreement and all rights of LICENSEE hereunder and all rights of any and all persons claiming under LICENSEE.

12.4 LICENSEE shall have the right to terminate this Agreement upon ninety (90) days notice prior to each anniversary date hereof.

12.5 Any termination of this Agreement shall be without prejudice to LICENSOR’s right to recover all amounts accruing to LICENSOR prior to such termination and cancellation. Except as otherwise provided, should this Agreement be terminated for any reason, LICENSEE shall have no rights, express or implied, under any Intellectual Property Right that is the subject matter of this Agreement. Upon termination, LICENSEE shall have the right to dispose of Products then in its possession and to complete existing contracts for such products, so long as contracts are completed within [\*\*] months from the date of termination. LICENSEE agrees to return to LICENSOR any Intellectual Property Rights and progeny, derivatives, developments or improvements thereof remaining in LICENSEE’s possession after [\*\*] months from the date of termination.

## **13. CERTIFICATE OF INSURANCE:**

13.1 LICENSEE agrees to carry and keep in force, at its expense, general and product liability insurance of not less than \$[\*\*] to cover liability for damages on account of bodily or personal injury or death to any person, or damage to property of any person; such insurance shall not be canceled for any cause without at least [\*\*] days prior written notice to Protein Sciences Corporation. At the time of execution of this Agreement, LICENSEE shall provide a certificate of insurance to LICENSOR.

## **14. GOVERNING LAW:**

Any disputes, controversies or claims which arise under, out of, in connection with, or relating to this Agreement shall be governed by and interpreted in accordance with the laws of the State of Connecticut, without regard to choice of law, and the parties agree that all disputes, controversies or claims which arise

under, out of, in connection with, or relating to this Agreement shall be brought in the Courts situated in the State of Connecticut for resolution thereof, and the parties

therefore submit to the exclusive jurisdiction of the State and Federal Courts situated in the State of Connecticut for resolution of all disputes, controversies or claims which arise under, out of, in connection with, or relating to this Agreement.

15. CAPTIONS:

The captions and Section headings of this Agreement are solely for the convenience of reference and shall not affect its interpretation.

16. SEVERABILITY:

Should any part or provision of this Agreement be held unenforceable or in conflict with the applicable laws or regulations of any jurisdiction, the invalid or unenforceable part or provision shall be replaced with a provision which accomplishes, to the extent possible, the original business purpose of such part or provision in valid and enforceable manner, and the remainder of the Agreement shall remain binding upon the parties hereto.

17. SURVIVAL:

- 17.1 The provisions of Sections 2.3, 4, 5, 6, 7, 8 and 14 shall survive the termination or expiration of this Agreement and shall remain in full force and effect.
- 17.2 The provisions of this Agreement which do not survive termination or expiration hereof (as the case may be) shall, nonetheless, be controlling on, and shall be used in construing and interpreting, the rights and obligations of the parties hereto with regard to any dispute, controversy or claim which may arise under, out of, in connection with, or relating to this Agreement.

18. AMENDMENT:

No amendment or modification of the terms of this Agreement, including any modification or amendment of this Section, shall be binding on either party unless reduced to writing and signed by an authorized officer of the party to be bound.

19. WAIVER:

No failure or delay on the part of a party in exercising any right hereunder will operate as a waiver of, or impair, any such right. No single or partial exercise of any such right will preclude any other or further exercise thereof or the exercise of any other right. No waiver of any such right will be deemed a waiver of any other right hereunder.

20. ENTIRE AGREEMENT:

This Agreement constitutes the entire agreement between the parties hereto respecting the subject matter hereof, and supersedes and terminates all prior agreements respecting the subject matter hereof, whether written or oral, and may be amended only by an instrument in writing executed by both parties hereto.

IN WITNESS WHEREOF, the parties hereto have caused this Agreement to be executed by their respective officers thereunto duly authorized to be effective as of the Effective Date.

Protein Sciences Corporation	Amsterdam Molecular Therapeutics
By: /s/ Manon M.J. Cox	By: /s/ Sander J.H. van Deventer
Manon M.J. Cox	Sander J.H. van Deventer
Chief Operating Officer	Chief Scientific Officer

APPENDIX I

1. Any rAAV Vector for the treatment of lipoprotein lipase deficiency disease in humans.

License Agreement - Non-Exclusive

(express+® cells)

AMENDMENT

Reference is made to a certain License Agreement dated as of March 22, 2007 by and between PROTEIN SCIENCES CORPORATION, whose principal place of business is at 1000 Research Parkway, Meriden, CT 06512 USA (hereinafter referred to as “LICENSOR”) and Amsterdam Molecular Therapeutics (AMT) B.V. whose principal place of business is at Meibergdreef 61, P.O. Box 22506,1100 DA Amsterdam, The Netherlands, (hereinafter referred to as “LICENSEE”), as amended (the “Agreement”).

Whereas:

On 10 May 2012, Amsterdam Molecular Therapeutics (AMT) B.V. changed its name to uniQure biopharma B.V.

LICENSOR and LICENSEE agree to amend the Agreement by adding to Appendix I thereof the following: “any rAAV Vector for the treatment of Acute Intermittent Porphyria disease in humans; any rAAV Vector for the treatment of Hemophilia B disease in humans”. The revised Appendix I is attached to this Agreement.

The Agreement is further amended as follows:

1. In Section 2.1 of the Agreement, the words “without the right to sublicense” shall be changed to “with the right to sublicense”, subject to the provision of Clause 4 of this Amendment.
2. Any Product LICENSEE proposes to add to Appendix I shall be automatically granted to LICENSEE as an exclusive license in the Territory subject to LICENSEE certifying to LICENSOR that it has acquired intellectual property covering such Product or it is actively developing such Product and subject further to a [\*\*]-day period during which LICENSOR shall notify LICENSEE if LICENSOR has, prior to LICENSEE’S proposed addition, (i) already granted a third party an exclusive license to such Product or competitive product or (ii) LICENSOR has an ongoing internal development program itself for such Product or a competitive product, in which case such Product will not be added to Appendix I. In the event that LICENSOR has granted a non-exclusive license to a third party for such Product or a competitive product, such Product will be added to Appendix I but designated as non-exclusive. After the lapse of the aforementioned [\*\*]-day period without notification to LICENSEE by LICENSOR, the Product concerned shall be automatically granted to LICENSEE as an exclusive license in the Territory.
3. Section 2.3 of the Agreement is amended as follows:
  - a. The portion of the last sentence shall be deleted starting with phrase “,provided that,”; and
  - b. Each time the phrase “Intellectual Property Rights” is used in such Section it is amended to read “Intellectual Property Rights or Technology”.
4. Section 8 of the Agreement is amended as follows:
  - a. The \$[\*\*] annual fee in respect of each Product in Appendix I shall not be payable after a total of \$[\*\*] has been paid in respect of such Product or such Product is no longer being developed;

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  - b. The maximum amount payable per annum in respect of Appendix I is \$[\*\*] per year regardless of the number of Products listed therein, and
  - c. In addition to the fees payable in respect of Products listed in Appendix I, in the event any Product that is or has been listed in Appendix I is being sold by LICENSEE in any market or is partnered with, licensed to or funded by another entity, LICENSOR shall be entitled to a fee of \$[\*\*] per year payable from the date such Product is first sold or such partnership, license or funding occurs and ending on the earlier of ten (10) years after the first commercial sale of the Product or the date such Product is no longer being sold in any market or such partnership, license or funding is terminated.
5. uniQure biopharma B.V. may sublicense under the Licensed Patent and the Technology and know how involving the Technology to the extent and only to the extent that such sublicense is reasonably necessary for a sublicensee to research, have researched, develop, have developed, manufacture, have manufactured, import, have imported, market, have marketed, offer for sale, sell and have sold a Product listed in Appendix 1 in the Territory. No general license to use the Technology by such sublicensee is implied or granted by the foregoing. Section 10 is amended to provide that LICENSEE may assign the Agreement in the event of a reorganization, merger, transfer, share exchange, consolidation, or sale or disposition of all or substantially all of the assets of LICENSEE.

In all other respects the Agreement is unchanged.

PROTEIN SCIENCES CORPORATION

uniQure biopharma B.V.

By: /s/Manon M.J. Cox

Manon M.J. Cox  
President and CEO  
Date: June 13, 2012

By: /s/ Piers Morgan

Name: Piers Morgan  
Title: CFO  
Date: June 13, 2012

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#### APPENDIX I

1. Any rAAV Vector for the treatment of lipoprotein lipase deficiency disease in humans. Exclusive.
  2. Any rAAV Vector for the treatment of Acute Intermittent Porphyria disease in humans. Exclusive.
  3. Any rAAV Vector for the treatment of Hemophilia B disease in humans. Exclusive.
-

Confidential Materials omitted and filed separately with the Securities and Exchange Commission. Double asterisks denote omissions.

**DATED 16 JUNE 2006**

**ACADEMISCH MEDISCH CENTRUM**

- and -

**BEHEERSMAATSCHAPPIJ DIENSTVERLENING EN DEELNEMING AZUA  
B.V.**

- and -

**AMSTERDAM VECTOR PRODUCTIONS B.V.**

- and -

**AMSTERDAM MOLECULAR THERAPEUTICS B.V.**

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**AGREEMENT**

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**THIS AGREEMENT** is made as of the day of 16 June, 2006

**BETWEEN:**

- (1) **ACADEMISCH MEDISCH CENTRUM**, whose principal address is Meibergdreef 9, 1105 AZ Amsterdam, the Netherlands ("**AMC**"); and
- (2) **BEHEERSMAATSCHAPPIJ DIENSTVERLENING EN DEELNEMING AZUA B.V.**, whose principal address is Meibergdreef 9, 1105 AZ Amsterdam, the Netherlands ("**BDDA**"); and

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- (3) **AMSTERDAM VECTOR PRODUCTIONS B.V.**, a closed limited liability company organized and existing under the laws of the Netherlands, with registered offices at Meibergdreef 61, 1105 BA Amsterdam, the Netherlands ("**AVP**").
  - (4) **AMSTERDAM MOLECULAR THERAPEUTICS B.V.**, a closed limited liability company organized and existing under the laws of the Netherlands, with registered offices at Meibergdreef 61, 1105 BA Amsterdam, the Netherlands ("**AMT**").

**RECITALS:**

- (A) AMC is a leading medical centre in the Netherlands which, amongst other activities, conducts research into the therapy, prophylaxis and diagnosis of various diseases and other conditions.
- (B) To the extent that AMC carries out activities relating to the results of its research it does so through BDDA.
- (C) AMT was founded in 1998 to further research, develop, manufacture and commercialise certain viral products and technologies which had arisen and which would arise at AMC. AMC has invested in AMT through BDDA and indirectly controls ninety-one point nine percent (91.9%) of the issued share capital of AMT before taking into account share option arrangements.
- (D) Various agreements have been made from time to time between AMC and AMT concerning the ownership and licensing of technical and scientific matter relating to the discovery, development, application and manufacture of viral products with therapeutic applications and resulting from the research activities of certain researchers employed by AMC within the framework of their activities as employees of AMC (whether such activities were conducted alone without collaboration with employees of AMT or in collaboration with employees of AMT) including a license agreement dated 22 January 2002, all such earlier agreements being called "Original IP Agreements" in this Agreement.

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- (E) Separately AMC and BDDA founded another company, AVP, to carry out the manufacture to GMP (as defined below) of certain therapeutic products. As AVP has not needed the capacity of the GMP facility and other manufacturing assets leased to it AVP has from time to time given AMT the right to use such GMP facility and other assets under the terms of various agreements ("Original AVP/AMT Agreements") to some of which AMC and/or BDDA have been a party. One implicit term of the Original AVP/AMT Agreements has been that AMT would, as employer of all AMT employees operating such GMP facility and other assets, own all Intellectual Property (as defined below) arising as a results of the activities of such employees.
  - (F) In addition to the Original IP Agreements and Original AVP/AMT Agreements there have from time to time been various other agreements made between any of AMC, BDDA and AVP on the one hand and AMT on the other hand concerning the business activities of AMT including financial, management and other agreements, all such other agreements being called "Other Original Agreements" in this Agreement.

- (G) In this Agreement Original IP Agreements, Original AVP/AMT Agreements and Other Original Agreements are collectively called “Original Agreements”.
- (H) In contemplation of a new fund raising being conducted by AMT pursuant to which there will be investment in AMT diluting BDDA’s shareholding in AMT the Parties (as defined below) are entering into this Agreement:
- a. to terminate all the Original Agreements; and
  - b. to assign to AMT any right, title or interest that AMC has in the Existing Patent Rights as hereafter defined; and
  - c. to set out certain financial terms agreed between the Parties; and
  - d. to permit AMT to use the GMP facility and other assets of AVP for a specified period and to set out the terms therefor.
- (I) For clarity this Agreement does not seek to regulate the following activities which it is intended shall be the subject of additional agreements between the relevant Parties to the extent that such activities will occur:

- a. following the Effective Date (as defined below) the access by AMT to the results of academic research in the Field (as defined below) by any employee of AMC and/or BDDA under any sort of pipeline arrangement; or
- b. following the Effective Date the sponsorship by AMT of any research to be conducted by employees of AMC and/or BDDA whether alone or in collaboration with employees or partners of AMT.

**NOW IT IS HEREBY AGREED** as follows:

## **1. DEFINITIONS AND INTERPRETATIONS**

1.1 In this Agreement in addition to the definitions in the Recitals the following definitions shall apply unless the context requires otherwise:-

“**AAV Vector**” - an adeno-associated virus vector composed of a transgene cassette with limited length (approximately 4.5 kb) flanked by an ITR DNA sequence.

“**Affiliate**” - any company, partnership or other business entity which controls, is controlled by or is under common control with either Party. For the purposes of this definition only, “control” refers to any of the following (a) the possession, directly or indirectly, of the power to direct the management or policies of an entity, whether through ownership of voting securities, by contract or otherwise; (b) ownership of fifty percent (50%) or more of the voting securities entitled to vote for the election of directors in the case of a corporation, or of fifty percent (50%) or more of the equity interest in the case of any other type of legal entity; (c) status as a general partner in any partnership, or any other arrangement whereby a Party controls or has the right to control the board of directors or equivalent governing body of a corporation or other entity.

“**Agreement**” - this document including any and all schedules, appendices and other addenda to it as may be added and/or amended from time to time in accordance with the provisions of this document.

“**AMC’s Ownership Interest**” - as defined in Section 3.1.

“**AMT Facility Manufacturing Know How**” - all manufacturing Know How that is in the possession of AMT as of the Effective Date and/or will come in the possession of AMT during the Rental Term, that is required by or useful for AVP to run the GMP Facility under GMP conditions without interruption as from the end of the Rental Term, including but not limited to all standard operation procedures related to the GMP Facility and all validations related to the equipment of the GMP Facility, but expressly excluding any Know How pertaining to the Products.

“**AVP Assets**” - all assets owned by AVP at the Effective Date set out in Schedule 1 that are physically present at the space the subject of the Rental Agreements and any replacements thereof under Section 9.2 and any other assets added to Schedule 1 by agreement between AVP and AMT from time to time.

“**Business Day**” - 9.00 am to 5.00 pm local time on a day other than a Saturday, Sunday, bank or other public holiday in the Netherlands.

“**Commercialisation**”, “**Commercialising**”, or “**Commercialise**” - all activities relating to the export, import, promotion, marketing, detailing, distribution, storage, handling, offering for sale and sale of FG Product.

“**Competent Authority**” - any national or local agency, authority, department, inspectorate, minister, ministry official, parliament or public statutory person (whether autonomous or not) of any government of any country having jurisdiction over either any of the activities contemplated by the Agreement or over the Parties.

“**Confidential Information**” - means (i) any Know How disclosed either directly or indirectly by one of the Parties hereto to the other and pertinent to the subject matter of this Agreement; and (ii) any other information coming into a Party’s possession relating to the other Party’s business as a result of this Agreement including relating to their business relationships, licensees, products, revenue flows, profitability, intellectual property or otherwise relating to their business affairs or finances which is either marked confidential at the time of disclosure by the other Party or is clearly confidential from its inherent nature.

**“Cover”** (including the variations such as “Covered”, “Coverage” or “Covering”) -the development, making, using or Commercialisation of a given product in a Commercialised form would infringe a Valid Claim of a Patent Right in the absence of a licence under such Patent Right. The determination of whether a product is so covered by a particular Patent Right shall be made on a country-by-country basis.

**“Documents”** - analyses, books, CD-ROM, charts, comments, computations, designs, discs, diskettes, files, graphs, ledgers, notebooks, paper, photographs, plans, records, recordings, reports, research notes, tapes and any other graphic or written data or other media on which Know How is permanently stored and other computer information storage means, and advertising and promotional materials of any nature whatsoever including preparatory materials for the same.

**“Effective Date”** - the date on which AMT completes the First Third Party Investment.

**“Existing Patent Rights”**- those Patent Rights jointly owned by AMT and AMC, details of which are set out in Schedule 2 and any Patent Rights subsequently derived therefrom.

**“Expert Determination”** - the Procedure set out in Schedule 3.

**“FG Products”** - LPL Products, IL-10 Products and Nash Products.

**“First Commercial Sale”**- for each FG Product, the first invoiced commercial sale by AMT, its Affiliates or sub-licensees to a third party in a country after grant of required Marketing Authorisation and pricing approval has been granted (if required in that country) by the appropriate Regulatory Authority. Sales for test marketing or clinical trial purposes do not constitute a First Commercial Sale.

**“First Third Party Investment”** - the first investment by a person or persons other than AMC or BDDA in AMT (whether in the form of equity or other equity instruments) or debt, which investment is greater than [\*\*] Euros (€[\*\*]).

**“First Rental Agreement”** - the rental agreement between AMC and AVP relating to certain space in Building R-Noord dated October 1, 2005, a copy of which is at Schedule 4 to this Agreement.

**“GMP”** - manufacture in accordance with Directive 91/412/EEC and Directive 2003/94/EC or any other applicable European Community Legislation or regulation as amended and applicable from time to time.

**“GMP Facility”** -the facility comprising the space the subject of the Rental Agreements and the AVP Assets.

**“IL-10 Product”** - a gene therapy product for the treatment of diseases and conditions caused by the deficiency or mutation of the IL-10 gene.

**“Insolvency Event”** - in relation to a Party, means any one of the following:

- (i) a resolution shall have been passed by that Party’s directors or by its shareholders to request a court to open insolvency proceedings as defined in Article 2 (a) of the European Insolvency Regulation; or
- (ii) a request to open such insolvency proceedings shall have been submitted by that Party to a court; or
- (iii) another person or entity who has the power to request the opening of insolvency proceedings as defined in Article 2(a) of the European Insolvency Regulation shall have submitted such request; or
- (iv) insolvency proceedings as defined in Article 2(a) of the European Insolvency Regulation have been opened with respect to that Party; or
- (v) a request has been filed for suspension of debts of AMT; or
- (vi) AMT, as a consequence of financial difficulties, makes any voluntary arrangement with its creditors outside a bankruptcy, suspension of payments or any other similar regulation; or
- (vii) an order is made or a resolution passed for the winding-up of AMT;

**“Know How”** - technical and other information which is not in the public domain, including information comprising or relating to concepts, discoveries, data, designs,

formulae, ideas, inventions, methods, models, assays, research plans, procedures, designs for experiments and tests and results of experimentation and testing (including results of research or development), processes (including manufacturing processes, specifications and techniques), laboratory records, chemical, pharmacological, toxicological, clinical, analytical and quality control data, trial data, case report forms, data analyses, reports, manufacturing data or summaries and information contained in submissions to and information from ethical committees and regulatory authorities. Know How includes Documents containing Know How, including but not limited to any rights including trade secrets, copyright, database or design rights protecting such Know How. The fact that an item is known to the public shall not be taken to preclude the possibility that a compilation including the item, and/or a development relating to the item, is not known to the public.

**“Legal Requirement”** - any present or future, law, regulation, directive, instruction, direction or rule of any Competent Authority or Regulatory Authority including any amendment, extension or replacement thereof which is in force from time to time.

**“LPL Product”** - a gene therapy product for the treatment of diseases and conditions caused by lipoprotein lipase deficiency or mutation.

**“Marketing Authorisation”** - any approval from a Regulatory Authority to market and sell a FG Product in any country including any form of reimbursement approval.

**“Nash Product”** - a gene therapy product for the treatment of non-alcoholic steatotic hepatitis.

**“Net Sales”** - the gross amounts invoiced by AMT, its Affiliates or sublicensees for all sales of FG Product less the following items to the extent that they are paid or actually allowed and are shown on the relevant invoice:

- (a) credits, allowances, discounts and rebates to, and charge backs from the account of, such customers for spoiled, damaged, out-dated, rejected, or returned FG Product;

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- (b) freight and insurance costs incurred in transporting such FG Product, to the extent such costs are itemized in the invoiced sales price;
- (c) cash, quantity, and trade discounts and other price reductions;
- (d) sales, use, value-added, and other direct taxes; and
- (e) customs duties, surcharges, and other governmental charges incurred in connection with the exportation or importation of such FG Product.

The transfer of FG Product by AMT or one of its Affiliates to another Affiliate shall not be considered a sale. In such cases Net Sales shall be determined based on the invoiced sale price by the Affiliate to the first third party trade purchaser, less the deductions allowed under this Section.

Upon the sale or other disposal of FG Product other than in a transaction generating revenues from or based on a sales price for the FG Product which sales price is either customary or would be reasonably expected in the country of sale, such sale, disposal or use shall be deemed to constitute a sale with the consideration for the sale being the consideration for the relevant transaction and constituting Net Sales hereunder or if the consideration is not a monetary amount a sale shall be deemed to have occurred for a price assessed on the value of whatever consideration has been provided in exchange for the supply. Disposal of FG Product for or use of FG Product in, clinical studies or as free samples shall not give rise to any deemed sale under this Section. For clarity, there shall be no limit on the quantity of FG Product which may be used in clinical trials but the quantity of FG Product to be given away as free samples shall be such quantities common in the industry for this sort of FG Product, which shall in any event not exceed [\*\*] units of FG Product per FG Product per field sales force representative per annum.

Disposal of FG Product for, or use of FG Product, in clinical studies or as free samples to be in quantities common in the industry for this sort of FG Product shall not give rise to any deemed sale under this Section.

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Such amounts shall be determined from the books and records of AMT and its Affiliates maintained in accordance with IFRS, consistently applied.

**“Party or Parties”** - AMC, BDDA, AVP and/or AMT.

**“Patent Rights”** - patent applications and patents and all foreign counterparts of them in all countries, including any renewals, re-examinations, continuations, continuations-in-part, patents of addition, extensions, (including patent term extensions,) reissues, substitutions, confirmations, and any equivalents of the foregoing in any and all countries of or to any of them, as well as any supplementary protection certificates and equivalent protection rights in respect of any of them.

**“Quarter”** - each period of three months ending on 31 March, 30 June, 30 September or 31 December and “Quarterly” shall be construed accordingly.

**“Regulatory Authority”** - any national, supranational, regional, state or local regulatory agency, department, bureau, commission, council or other governmental agency including the FDA, in any country involved in the grant of Marketing Authorisation.

**“Rental Agreements”** - the First Rental Agreement and the Second Rental Agreement. For clarity, the Rental Agreements are not Original Agreements.

**“Rental Term”** - as defined in Section 7.1

**“Second Rental Agreement”** - the rental agreement between BDDA and AVP relating to certain space in Building R-Zuid, a copy of which is at Schedule 5 to this Agreement.

**“Total Available Production Capacity”** - the total number of project weeks per Use Year available comprised of clean room activity and related preparatory and post clean room activity in the space subject to the First Rental Agreement and the space subject to the Second Rental Agreement together (including production hours to be used by AMT itself and/or third parties) being [\*\*] project weeks per Use Year.

**“Use Year”** - as defined in Section 11.1:

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**“Valid Claim”** - either:

- (a) a claim of an issued and unexpired patent included within Patent Rights, which has not been disclaimed or held permanently revoked, unenforceable or invalid by a decision of a court or other governmental agency of competent jurisdiction, unappealable or unappealed within

the time allowed for appeal, and which has not been admitted to be invalid or unenforceable through reissue or disclaimer or otherwise; or

- (b) a claim of a pending patent application included within Patent Rights which claim has been filed and has been prosecuted in good faith for not more than [\*\*] years and has not been abandoned or finally disallowed without the possibility of appeal or refiling of the application.

## **2. TERMINATION OF ORIGINAL AGREEMENTS**

- 2.1 The Parties agree that, with effect from the Effective Date, all of the Original Agreements shall terminate including any provisions of them which expressly or impliedly are stated to survive such termination.
- 2.2 Subject to Section 2.4 each Party hereby releases and discharges the others from all their obligations and liabilities under the Original Agreements as at the Effective Date.
- 2.3 Subject to Section 2.4 each Party waives any and all claims of any nature whatsoever and howsoever arising (whether now or in the future and whether presently known or unknown) and including, without limitation, for payment or otherwise which it may have against another under the Original Agreements.
- 2.4 At the Effective Date there is a [\*\*] Euro (€[\*\*]) loan from BDDA to AMT outstanding. Repayment of this loan shall be deferred until a “Successful Exit” being either: (i) the occurrence of a future liquidity event allowing a successful exit for the investors in AMT that realises for such investors an investment multiple of at least [\*\*] times their original investment; or (ii) the becoming effective of a fully underwritten initial public offering of the shares in AMT at a price per share equal to or greater than [\*\*] times the original

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subscription price (being two point nine four six Euros (€2.946) per share) and with gross proceeds in excess of [\*\*] Euros (€[\*\*]). It is acknowledged by the Parties that as part of the First Third Party Investment the subscription agreement may contain a more detailed definition of “Successful Exit” in relation to the repayment of the loan compared to that set out above, and if it does upon execution of such subscription agreement the Parties shall amend the definition of Successful Exit set out above so that it reads the same as the definition of Successful Exit set out in the subscription agreement.

## **3. ASSIGNMENT**

- 3.1 AMC hereby transfers and assigns its right, title or interest in the Existing Patent Rights to AMT (“AMC’s Ownership Interest”), including:
- 3.1.1 the right to sue for and recover damages and other remedies in respect of any infringement of the Existing Patent Rights or other acts carried out by another person within the scope of the claims of any published specifications of any of the Existing Patent Rights which may have occurred before the Effective Date; and
- 3.1.2 the right to apply for, prosecute and obtain patent or similar rights or protection in respect of any of the inventions the subject matter of the Existing Patent Rights in any country of the world (including the right to claim priority from the Existing Patent Rights).
- 3.2 AMC agrees that it will at the request of AMT or AMT’s patent attorneys do and execute or arrange for the doing and executing, of each necessary act, document or thing to implement the assignment and transfer the subject of Section 3.2 including those things necessary to effect the assignment at the patent office in such country on a country by country basis in a form of assignment approved by AMT’s external patent counsel.
- 3.3 Following the Effective Date, AMT and AMC may (but are not obliged to) enter into further agreements relating to the research and development of FG Products or other products Covered by the Existing Patent Rights (“Research Agreements”). The terms of

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such Research Agreements shall be negotiated by AMC and AMT in good faith on a case by case basis.

- 3.4 AMC does not grant any warranties or representations regarding the validity or enforceability of the Existing Patent Rights. Nor does it give any warranty or representation that the use of the Existing Patent Rights would not infringe third party’s rights. AMC shall not be held liable by AMT in the event of such infringements and shall be indemnified and held harmless by AMT from claims in relation to such infringement.
- 3.5 AMT shall use commercial reasonable and diligent efforts directed to obtain Marketing Authorisation for the FG Products and to commercialise the FG Products.

## **4. PAYMENTS TO AMC**

- 4.1 In consideration of the assignment of AMC’s Ownership Interest in the Existing Patent Rights, AMT has agreed to pay AMC royalties at the rate of [\*\*] per cent ([\*\*]%) Net Sales on sales of FG Products. Such royalties shall be payable on an FG Product by FG Product basis for so long as there are Valid Claims of Existing Patent Rights covering that FG Product in the country of sale or, if no Valid Claim exists in such country, for as long as AMT, its Affiliates and/or its sublicensees have a monopoly position in such country, whereby “monopoly” for the purpose of this clause means that in such country there is a juridical basis (i.e. granting of Orphan Drug Status) pursuant to which AMT has a position as if Valid Claims would exist in such country.

## **5. PAYMENT TERMS**

- 5.1 AMT shall make the payments due to AMC under Section 4.1 in Euros at Quarterly intervals. Within [\*\*] days of the end of each Quarter after launch of a FG Product in any country, AMT shall pay all monies due to AMC under Section 4.1. Each royalty payment shall be accompanied by a report summarising the Net Sales of each FG Product on a country-by-country basis during the Quarter, the currency conversion rate, if applicable (see Section 5.2), the taxes withheld, if any, and the total royalty payments due.



- 5.2 When for the purpose of calculating royalties under Section 4.1. conversion from any foreign currency to Euros shall be required, such conversion shall be made as follows. When calculating Net Sales, the amount of such sales in any particular foreign currency for a Quarter shall be converted into Euros, using the average Quarterly rate of exchange for such currency to Euros.
- 5.3 Payments made under Sections 4.1 shall be made free and clear of and without deduction or deferment in respect of any disputes or claims whatsoever and/or as far as is legally possible in respect of any taxes imposed by or under the authority of any government or public authority. Any tax which AMT is required to pay or withhold with respect of royalty payments to be made to AMC hereunder shall be deducted from the amount otherwise due provided that, in regard to any such deduction, AMT shall give AMC such assistance, which shall include the provision of such documentation as may be required by revenue services, as may reasonably be necessary to enable AMC to claim exemption therefrom or obtain a repayment thereof or a reduction thereof and shall upon request provide such additional documentation from time to time as is needed to confirm the payment of tax.
- 5.4 AMT shall prepare an annual statement which shall show for each calendar year all monies due to AMC under this Agreement. This statement shall be submitted to AMC within [\*\*] days of the end of a calendar year and shall include a copy of all statements received from licensees relating to the calendar year in question. If AMC gives notice to AMT within [\*\*] days of the receipt of any such statement that it does not accept the same, that statement shall be certified by an independent accountant appointed by agreement between AMT and AMC or, in default of agreement within [\*\*] days, appointed by the President for the time being of the Netherlands Instituut van Registeraccountants (NIVRA). AMT shall (subject to the independent accountant agreeing to maintain the confidentiality of the books and records save insofar as is necessary for the property reporting to AMT and AMC) make available to the independent accountant all books and records required for the purpose of that certification and the statements so certified shall be final and binding between the Parties. The cost of the certification shall be the responsibility of AMT if the statement is shown

to have underestimated the monies payable to AMC by more than [\*\*] per cent ([\*\*]%) and the responsibility of AMT otherwise. Any outstanding payments due to AMC which are identified as a result of carrying out the investigation shall be paid to AMC immediately. There shall be no more than [\*\*] by an independent accountant in relation to any one annual statement.

- 5.5 All payments to AMC under the terms of the Agreement are expressed to be exclusive of VAT. All payments made to AMC under the Agreement shall be made in Euros by direct transfer to such bank account as AMC may notify AMT from time to time.
- 5.6 If AMT fails to make any payment to AMC hereunder by the due date for payment without prejudice to any other right or remedy available to AMC, AMC shall be entitled to charge AMT interest (both before and after judgement) on the amount unpaid at the rate of [\*\*] calculated on a daily basis until payment in full is made.

## **6. RE-ASSIGNMENT OF EXISTING PATENT RIGHTS**

- 6.1 If, following the Effective Date, (i) there is a family of Existing Patent Rights which Cover a specific FG Product (“**Patent Family**”), and (ii) the board of AMT decides to cease developing and Commercialising the FG Product Covered by that Patent Family in each of Europe (considered as a whole), the United States of America and Canada whilst there are Valid Claims of Existing Patent Rights in the Patent Family in question in such country, then AMT shall notify AMC of such board decision and shall forthwith upon AMC’s request, re-assign to AMC free of charge AMC’s Ownership Interest in such Patent Family.
- 6.2 AMT hereby appoints AMC as its attorney to effect on its behalf any re-assignment of the AMC Ownership Interest under Section 6.1 which AMT has failed to make to AMC within [\*\*] days upon AMC’s request as referred to in Section 6.1, with the right but not the obligation to do any and all acts and things necessary to effect unconditionally such re-assignment including the right for AMC to execute all deeds, documents or instruments and swear any oaths or declarations in the name of and on behalf of AMT necessary for such purpose. AMC’s appointment as attorney under this Section 6.2 is given to secure AMC’s Ownership Interest in the Existing Patent Rights and to secure the

performance of AMT’s obligations to re-assign the AMC Ownership Interest in the event of termination and such appointment shall be perpetual and irrevocable, notwithstanding AMT entering into liquidation, being wound-up or dissolved or having a receiver, manager, administrator, administrative receiver or similar person appointed over any of its assets.

- 6.3 Subject to the provisions of Section 6.1 AMC’s only remedy for a material breach of Sections 3 to 5 shall be a claim for damages.

## **7. USE BY AMT OF GMP FACILITY**

- 7.1 With effect from Effective Date AVP hereby grants to AMT exclusive access to the GMP Facility and the exclusive right to use the same for the period from the Effective Date to December 1, 2010 (“**Rental Term**”) on the terms of Sections 8 to 11 of this Agreement. AMC and BDDA consent and agree to such grant by AVP.
- 7.2 AVP gives no warranty or representation to AMT that by using the GMP Facility AMT will be able to manufacture products to GMP.

## **8. RENTAL AGREEMENTS**

- 8.1 AMT acknowledges and agrees that it has seen a copy of the Rental Agreements and undertakes to comply with the terms thereof as if they formed part of this Agreement and in particular AMT will reimburse AVP all rental payments made by AVP to AMC and BDDA respectively pursuant to the Rental Agreements within [\*\*] days of receipt of an invoice therefore from AVP which attaches the corresponding AMC or BDDA invoice to AVP.

- 8.2 AMC and AVP shall not amend or vary the First Rental Agreement nor shall BDDA and AVP amend the Second Rental Agreement in either case during the Rental Term without AMT's consent save where such amendment is necessary to comply with a Legal Requirement.

## 9. PAYMENT FOR THE USE OF AVP ASSETS

- 9.1 In consideration of the use of the AVP Assets by AMT, AMT shall pay to AVP its depreciation costs on the AVP Assets in the amounts and on or before the dates set forth in Schedule 1A. Subject to Section 9.3, each AVP Asset shall become the sole property of AMT upon the payment of the last amount set forth in Schedule 1 A.
- 9.2 AMT shall have the right to replace any particular AVP Asset and dispose of the original at its own cost and expense and, subject only to Section 9.3, AMT shall own such replacement AVP Asset. For clarity, replacing an original AVP Asset shall not relieve AMT of its payment obligations in respect of that AVP Asset under Section 9.1 (if any).
- 9.3 Should any AVP Asset (including any replacement AVP Asset acquired on the terms of Section 9.2) be one which is a fixed, immovable asset, such that removing that asset from the GMP Facility upon termination or expiration of the Rental Term may cause material damage to the GMP Facility (a "Fixed Asset"), that asset shall be and shall remain the property of AMC. AMC hereby authorizes AMT to use the Fixed Assets for all purposes necessary for AMT to operate the GMP Facility. Following termination or expiration of the Rental Term AMC may elect that AMC and/or AVP should continue using the Fixed Assets. If AMC so elects it shall notify AMT of this decision, whereupon AMC shall pay or shall procure the payment to AMT of a commercially reasonable amount for the use of such Fixed Assets taking into account: (i) the amount paid for the original Fixed Asset by AMT to AVP under Section 9.1, (ii) the cost of any replacement asset acquired by AMT pursuant to Section 9.2 (if any); (iii) the age of the Fixed Asset upon the expiration or termination of the Rental Term; and (iv) the estimated remaining life of the Fixed Asset. Any dispute between the Parties as to the size of this payment shall be referred to Expert Determination. In the event that upon the expiration or termination of the Rental Term AMC does not elect to continue to use a Fixed Asset the Parties shall discuss alternative methods for disposing of that Fixed Asset.

## 10. OPERATIONS

- 10.1 AMT shall only use the GMP Facility for the purpose of the research, development and manufacture of gene and cell therapy products including FG Products, as well as the support functions related to this purpose.
- 10.2 AMT shall maintain the GMP Facility (including the AVP Assets) in good condition and shall bear the sole risk for any damage, loss, destruction or theft thereof.
- 10.3 AMT shall have and maintain such type and amounts of insurance covering the GMP Facility as is normal and customary in the biotechnology industry generally for persons similarly situated, and shall upon request provide AVP with a copy of its policies or certificates of insurance (as available) in that regard, along with any amendments and revisions thereto.
- 10.4 AMT will be responsible for obtaining all required licenses, consents and other regulatory permissions necessary for the operation of the GMP Facility.

## 11. AMC'S USE OPTION

- 11.1 AMC has the right on an annual basis expiring November 30, 2007 and each subsequent November 30 during the Rental Term ("Use Year") to use:

- 11.1.1 for the [\*\*] Use Years from [\*\*], up to no more than [\*\*] percent ([\*\*]%) of AMT's Total Available Production Capacity in the GMP Facility; or
- 11.1.2 for the [\*\*] Use Years from [\*\*], up to but no more than [\*\*] percent ([\*\*]%) of AMT's Total Available Production Capacity in the GMP Facility.

For clarity, it is declared and agreed that AMC shall have no right for its employees to have access to the GMP Facility, the right of use being a right to have AMT employees operate the GMP Facility on AMC's behalf.

- 11.2 If AMC wishes to exercise this right of use for any Use Year, AMC must notify AMT in writing of the exercise of this right no later than [\*\*] of the year preceding the Use Year in which it wishes to use that production capacity. For clarity, should AMC not exercise

its option for a particular Use Year as referred to above AMC shall owe AMT no fee and AMT shall be free to use Total Available Production Capacity in that Use Year for its own needs. If AMC has given notice to use then no later than [\*\*] prior to the commencement of the Use Year in question AMC and AMT shall agree the precise use for which AMC requires AMT to operate the relevant capacity and the precise capacity to be used (not being in excess of the aforesaid percentages) and agreed deliverables to be supplied to AMC from such use and the price therefor to be paid to AMT by AMC calculated in accordance with Section 11.3. The agreed terms of such arrangement shall be confirmed by AMC to AMT by notice in writing, such notice ("Agreed Use Notice") shall be given by no later than the [\*\*] prior to the commencement of the Use Year in question. AMT shall execute the work set out in the Agreed Use Notice for or on behalf of AMC in compliance with the protocols submitted by AMC to AMT for such work and in compliance with all applicable GMP regulations. Should AMC have given an Agreed Use Notice as referred to above by the [\*\*] deadline and should AMC then not use any or all of the production capacity indicated in its Agreed Use Notice AMC shall in any event pay AMT the fee for such capacity calculated set out in the Agreed Use Notice. If AMC fails to give AMT an Agreed Use Notice by the [\*\*] deadline AMC shall owe AMT no fee and AMT shall be free to use Total Available Production Capacity in that Use Year for its own needs.

- 11.3 The price that AMT will charge to AMC will be calculated by reference to the actual costs (including but not limited to the costs of the personnel and materials used by AMT) incurred by AMT to perform the work for AMC in accordance with GMP and the protocols submitted by AMC, plus [\*\*] percent ([\*\*]%) thereof. AMT will invoice AMC for such costs at the end of the month in which such costs are incurred. AMC will make payment for such sums within [\*\*] days of receipt of an invoice. All payment to AMT under the terms of this Agreement are expressed to be exclusive of VAT, and shall be made in Euros by direct transfer to such bank account as AMT may notify AMC from time to time. If AMC fails to make payment to AMT hereunder by the due date for payment then without prejudice to any other rights or remedy available to AMT, AMT shall be entitled to change AMC interest (both before and after judgement) on the amount unpaid at the rate of [\*\*] calculated on a daily basis until payment in full is made.

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## 12. TERM AND TERMINATION

- 12.1 The provisions of Section 7 to 11 of this Agreement will be effective for the Rental Term unless terminated earlier pursuant to Section 12.2, 12.3 or 12.6.
- 12.2 AVP shall be entitled to terminate the provisions of Section 7 to 11 of this Agreement by written notice to AMT with immediate effect if AMT:
- 12.2.1 commits a material breach of Section 7 to 11 and, where the breach is capable of remedy, has failed to remedy such breach within [\*\*] days of written notice requiring remediation; or
  - 12.2.2 suffers an Insolvency Event.
- 12.3 AMT shall be entitled to terminate the provisions of Section 7 to 11 of this Agreement at any time prior to the end of the Rental Term without reason upon giving twelve (12) months written notice to AVP.
- 12.4 Upon the expiry of the Rental Term or the termination of Sections 7 to 11 by AVP for cause pursuant to Section 12.2 or a termination of Sections 7 to 11 by AMT under Section 12.3, AMT will promptly vacate the GMP Facility, leaving the GMP Facility and AVP Assets in good condition and in conformity with then applicable GMP requirements and in such a state that AMC or AVP is able to run the GMP Facility as from the expiry or termination of the Rental Term without interruption. In order to achieve such smooth transition of the GMP Facility from AMT to AMC and/or AVP, AMT is willing, upon AMC/AVP's request to render reasonable assistance to AMC and/or AVP (which request may be made by AMC/AVP prior to the expiration or termination of the Rental Term to allow AMC/AVP sufficient time for such transition). Reasonable assistance will include providing reasonable GMP instruction to AMC/AVP personnel and/or assisting AMC/AVP in writing the Standard Operating Procedures AMC/AVP requires in order to operate the GMP Facility and also will include reasonable assistance as to the assignment of any permits and licenses required by AMC/AVP to run the GMP Facility.

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- 12.5 Upon the termination or expiry of Sections 7 to 11 for any reason, subject as otherwise provided in this Agreement and to any rights or obligations which have accrued prior to termination of the provisions of this Agreement which are expressed to survive termination, no Party shall have any further obligation to another under Sections 7 to 11.
- 12.6 Upon the termination of either Rental Agreement the provisions of Sections 7 to 11 in relation to that part of the GMP Facility and the AVP assets sited there shall also terminate, provided always that if such termination of a Rental Agreement occurs through no fault of AMT then AMC and BDDA (as applicable) will at that time enter into a direct agreement with AMT with regard to the relevant part of the GMP Facility on financial and other terms equivalent to those set out in the relevant Rental Agreement.

## 13. CONFIDENTIALITY

- 13.1 Each of the Parties undertakes to:
- 13.1.1 keep the Confidential Information secret and confidential and not to disclose it to any third party without the other Party's prior written consent save as expressly or impliedly permitted under the Agreement;
  - 13.1.2 use the Confidential Information only for the purposes envisaged under this Agreement and not to use the same for any other purpose whatsoever;
  - 13.1.3 ensure that only those of its officers, consultants, employees (including without limitation directors), Affiliates, licensees and other third parties who are directly concerned with this Agreement have access to the Confidential Information on a strictly applied "need to know" basis and are informed of the secret and confidential nature of it;
  - 13.1.4 keep the Confidential Information separately identifiable and safe and secure at all times in accordance with good industry practice; and
  - 13.1.5 clearly identify the Confidential Information as confidential.
- 13.2 The obligations of confidentiality shall not extend to any Confidential Information which:

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- 13.2.1 is or becomes generally available to the public otherwise than by reason of breach by the recipient of such Confidential Information (the "**Receiving Party**") of such a confidentiality obligation; or
- 13.2.2 is known to the Receiving Party and is at its free disposal at the time of its disclosure to the Receiving Party (having been generated independently by the Receiving Party or a third party in circumstances where it can be shown that it has not been derived from access to the

Confidential information of the Party disclosing such information (the “**Disclosing Party**”)) provided always that this provision shall not apply to the Original Intellectual Property and the obligations on AMC, BDDA and AVP in relation to the same; or

- 13.2.3 is subsequently disclosed to the Receiving Party without obligations of confidentiality by a third party owing no such obligations to the Disclosing Party in respect of that Confidential Information; or
- 13.2.4 is required by law to be disclosed (including as part of any regulatory submission or approval process) and then only when prompt written notice of this requirement has been given to the Disclosing Party so that it may, if so advised, seek appropriate relief to prevent such disclosure provided always that in such circumstances such disclosure shall be only to the extent so required and shall be subject to prior consultation with the Disclosing Party with a view to agreeing timing and content of such disclosure.
- 13.3 The Parties understand that remedies in damages may be inadequate to protect against any breach of any of the provisions of a confidentiality obligation by either Party or their employees, officers or any other person acting in concert with it or on its behalf. Accordingly, each Party shall be entitled to seek the granting of interim and final injunctive relief by a court of competent jurisdiction in the discretion of that court against any action that constitutes any breach of such an obligation.
- 13.4 The Parties agree that these obligations of confidentiality shall continue to apply until the Confidential Information is no longer confidential.

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## 14. MISCELLANEOUS

- 14.1 No Party shall without the prior written consent of the others, such consent not to be unreasonably withheld, assign the benefit and/or burden of this Agreement, provided, however, that either Party may assign this Agreement or any part of its rights and obligations hereunder to an Affiliate or to any company with which such Party may merge or consolidate, or to which it may transfer all or substantially all of its assets to which this Agreement relates, without obtaining the consent of the other Party provided always that such Affiliate or company undertakes in writing to the other Party to be bound by the terms of this Agreement.
- 14.2 If a Party is unable to perform any of its obligations under this Agreement due to an event of force majeure as determined by the common law such Party shall be excused such performance (but only such performance) during the period of such force majeure event.
- 14.3 The validity, construction and interpretation of this Agreement and any determination of the performance which it requires shall be governed by Netherlands law. All disputes between the Parties arising out of the circumstances and relationships contemplated by this Agreement including disputes relating to the validity, construction or interpretation of this Agreement, and including disputes relation to pre-contractual representations, which result in any action or proceeding shall be subject to the jurisdiction of the Netherlands Courts, District of Amsterdam.
- 14.4 Save as expressly provided in this Agreement nothing herein takes away from a Party or constitutes a waiver by a Party of any of its rights or remedies under common law, statute or otherwise.
- 14.5 This Agreement constitutes the entire agreement and understanding between the Parties and supersedes all prior oral or written understandings, arrangements, representations or agreements between them relating to the subject matter of this Agreement provided that this does not remove any right of action by either Party in respect of any fraudulent misrepresentation, fraudulent concealment or other fraudulent action.

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- 14.6 Only the Parties and their successors and permitted assignees shall have the right to enforce any provision of this Agreement and no other person shall have any rights to enforce a term of this Agreement which confers a benefit on that person.
- 14.7 No Party shall be liable to any other Party, its Affiliates or licensees in contract, tort, negligence, breach of statutory duty or otherwise for any loss, damage, cost or expense of an indirect or consequential nature (including any economic loss or other loss of turnover, profits, business or goodwill) arising out of or in connection with this Agreement or the subject matter of this Agreement.
- 14.8 All formal notices to be given pursuant to this Agreement shall be in writing and shall be delivered by hand to the address of the Parties set out above (or such other address as may be notified by a Party to the other from time to time) with a confirmation copy being sent by post. Notices shall be deemed to have been received at the time of delivery by hand.
- 14.9 The activities of the Parties contemplated pursuant to this Agreement shall not constitute a partnership and neither party has the authority to bind the other Party in any way except provided in this Agreement.
- 14.10 Each Party shall bear its own legal costs and other expenses incurred in the negotiation, preparation, execution and implementation of this Agreement.
- 14.11 Any press releases to be made by either Party relating to this Agreement will require the approval of the other Party.
- 14.12 This Agreement may be executed in any number of counterparts, each of which shall be deemed an original and all of which taken together shall be deemed to constitute one and the same instrument. An executed signature page of this Agreement delivered by facsimile transmission shall be as effective as an original executed signature page.

IN WITNESS WHEREOF the Parties have executed this Agreement the day and year first above written.

SIGNED by \_\_\_\_\_ ) /s/ illegible

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for and on behalf of )  
**ACADEMISCH MEDISCH CENTRUM )**  
  
SIGNED by )  
  
for and on behalf of )  
  
**BEHEERSMAATSCHAPPIJ DIENST- ) /s/ illegible**  
  
**VERLENING EN DEELNEMING )**  
  
**AZUA B.V. )**  
  
SIGNED by )  
  
for and on behalf of )  
  
**AMSTERDAM VECTOR ) /s/ J.M. ter Riet**  
  
**PRODUCTIONS B.V. )**  
  
SIGNED by )  
  
for and on behalf of )  
  
**AMSTERDAM MOLECULAR ) /s/ illegible**  
  
**THERAPEUTICS B.V. )**

SCHEDULE 1

AVP ASSETS

SCHEDULE1A

SCHEDULE OF DEPRECIATION PAYMENTS FOR THE AVP ASSETS

Confidential Materials omitted and filed separately with the Securities and Exchange Commission. A total of seven pages were omitted. [\*\*]

SCHEDULE 2

EXISTING PATENT RIGHTS

Title	Stage/Territory	Official No.	Date filed	Earliest priority date
[**]	[**]	[**]	[**]	[**]
	[**]	[**]	[**]	[**]
	[**]	[**]	[**]	[**]
	[**]	[**]	[**]	[**]
[**]	[**]	[**]	[**]	[**]
	[**]	[**]	[**]	[**]
	[**]	[**]	[**]	[**]
[**]	[**]	[**]	[**]	[**]

SCHEDULE 3

EXPERT DETERMINATION

1.
- Any matter or dispute to be determined by an expert under this Agreement (“Expert”) shall be referred to a person suitably qualified to determine that particular matter or dispute who shall be nominated jointly by the Parties or, failing agreement between the Parties within [\*\*] Business Days of a written

request by either Party to the other seeking to initiate the Expert's decision procedure, either Party may request the President for the time being of the Association of the Dutch Pharmaceutical Industry, NEFARMA, or any successor body to it to nominate the Expert.

2. The Parties shall with [\*\*] days of the appointment of the Expert meet with him/her in order to agree a program for written and oral submissions.
3. In all cases the terms of appointment of the Expert by whomsoever appointed shall include:
  - 3.1 a commitment by the Parties to share equally the Expert's fee;
  - 3.2 a requirement on the Expert to act fairly as between the Parties and according to the principles of natural justice;
  - 3.3 a requirement on the Expert to hold professional indemnity insurance both then and for [\*\*] years following the date of his/her determination;
  - 3.4 a commitment by the Parties to supply to the Expert the submissions the subject of paragraph 2 and all such assistance, documents and information as he/she may require for the purpose of his or her determination.
  - 3.5 a commitment by the Parties that all negotiations connected with the dispute shall be conducted in confidence and without prejudice to the rights of the Parties in any future proceedings.
4. The Expert shall give a written decision which shall contain a factual analysis, his/her conclusions and the reasons for his conclusions.

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5. The Expert's decision shall be final and binding on the Parties (save in the case of negligence or manifest error).
6. The Parties expressly acknowledge and agree that they do not intend the reference to the Expert to constitute an arbitration within the scope of any arbitration legislation, the Expert's decision is not a quasi-judicial procedure and the Parties shall have no right of appeal against the Expert's decision provided always that this shall not be construed as waiving any rights the Parties might have against the Expert for breaching his/her terms of appointment or otherwise being negligent.

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#### **SCHEDULE 4**

##### **FIRST RENTAL AGREEMENT**

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#### **SCHEDULE 5**

##### **SECOND RENTAL AGREEMENT**

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#### **Draft Side Letter to be typed on headed notepaper of AMT**

Academisch Medisch Centrum  
Meibergdreef 9,  
1105 AZ  
Amsterdam  
The Netherlands

[date]

Dear Sirs,

**Agreement made between us dated [ ] 2006**

We refer to the Agreement made between ourselves, yourselves, BDDA and AVP, dated [ ] 2006 ("the Agreement") and in particular to Section 11.2 of the Agreement. We confirm that we have agreed between us that for the balance of the first Use Year to November 30, 2006, we will use on your behalf the following of our Total Available Production Capacity on the following terms:

1. Capacity being used - [ ]
2. Deliverables to be supplied to AMC
3. Price [ ]

Kindly signify your agreement to the terms set out in this Side Letter by signing the attached copy of this Side Letter and returning it to us.

Yours sincerely

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For and on behalf of Amsterdam Molecular Therapeutics

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We acknowledge and agree the terms set out in this Side Letter for and on behalf of AMC

Confidential Materials omitted and filed separately with the Securities and Exchange Commission. Double asterisks denote omissions.

## LICENSE AGREEMENT

Between:

Aventis Pharma S.A.

20 avenue Raymond Aron

92160 Antony

France

(hereinafter referred to as "AVENTIS")

And,

Amsterdam Molecular Therapeutics B.V.

Meibergdreef 61

1105 BA Amsterdam

The Netherlands

(hereinafter referred to as "AMT").

## PREAMBLE

WHEREAS AVENTIS is the owner of the certain patents relating to the preparation and use of recombinant viruses in gene therapy.

WHEREAS the University of British Columbia ("UBC") is co-owner or is entitled to be co-owner of such patents but AVENTIS is entitled to exploit the invention directly or indirectly through third parties.

WHEREAS AMT is interested to get a license in order to exploit the invention in the Field and in the Territory (as more fully described below), under the terms and conditions defined therein.

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NOW, THEREFORE, the parties agree as follows:

### 1. DEFINITIONS

- 1.1 "Affiliate" shall mean, with respect to AVENTIS, any company which controls, is controlled by or is under common control with AVENTIS, and shall mean, with respect to AMT, any company which controls, is controlled by or is under common control with AMT. Control shall mean the direct and/or indirect legal or beneficial ownership of more than fifty (50) percent of the voting stock of such company.
- 1.2 "Effective Date" shall mean the date the License Agreement comes into force being the date that the last party signs the same.
- 1.3 "Field of Use" shall mean indications of Lipoprotein Lipase (LPL) deficiency also referred to as Type I or V hyperlipoproteinemia.
- 1.4 "License Agreement" shall mean this license agreement between AVENTIS and AMT dated the Effective Date.
- 1.5 "Net Sales" shall mean the gross sales invoiced for the Product by AMT and its Affiliates to third parties in the Territory less: (i) quantity and/or cash discounts, allowances actually allowed or given, and transportation and insurance costs for shipments to customers; (ii) credits or refunds actually allowed for returned Product; (iii) compulsory payments and rebates, accrued, paid or deducted pursuant to agreements (including but not limited to managed care agreements) or governmental regulations and (iv) sales and other excise taxes directly related to that sale, to the extent that such items are included in the gross invoice price (but not including taxes assessed against the income derived from such sale).
- 1.6 "Patents" shall mean the patents or patent applications listed in Annex 1 hereto and their divisional, reexamination, continuation-in-part, or extension including supplemental protection certificate, which are or will be owned by AVENTIS and UBC and which relate to the preparation and use of recombinant viruses in gene therapy.

- 1.7 "Product" shall mean a gene therapy product containing a recombinant virus with a nucleic acid coding for a lipoprotein lipase (LPL) developed, used and/or commercialised by or for AMT or its affiliates where the activity in relation to such product occurs in one of the countries of the Territory and is covered in whole or in part by a Valid Claim within the Patents in that country.
- 1.8 "Territory" shall mean the countries listed in Annex 1.



1.9 “Valid Claim” means any claim of any issued or granted and unexpired Patent which has not been held unenforceable, unpatentable or invalid by a court or other governmental agency of competent jurisdiction in an unappealed or unappealable decision, and which has not been disclaimed or admitted to be invalid or unenforceable through reissue or disclaimer award in an interference proceeding, or final rejection in a re-examination or re-issue proceeding, and which has not lapsed, been withdrawn or otherwise gone abandoned.

## 2. GRANT - CONDITION PRECEDENT

2.1 AVENTIS hereby grants to AMT and AMT accepts from AVENTIS an exclusive license right under the Patents to develop, use, make, have made, sell or offer to sell Product in the Field of Use and in the Territory.

2.2 The license shall be non-transferable and non-assignable except for Affiliates of AMT.

2.3 The grant of rights hereunder is subject to, and shall only come into force upon the unconditional withdrawal by AMT of the opposition filed by AMT on June 3<sup>rd</sup> 2003 against [\*\*].

## 3. CONSIDERATION

3.1 In consideration of the rights granted by AVENTIS to AMT under this License Agreement AMT undertakes to effect the following payments:

(a) Ten thousand Euros (10.000 €) upon signature of the Agreement;

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(b) Fifty thousand Euros (50.000 €) upon [\*\*];

(c) Seventy-five thousand Euros (75.000 €) at the [\*\*];

(d) Royalties of [\*\*] percent ([\*\*]%) of Net Sales of Product in the Territory as long as total cumulative Net Sales of Product are below [\*\*] Euros ([\*\*]€);

(e) Royalties of [\*\*] percent ([\*\*]%) of Net Sales of Product in the Territory as soon as total cumulative Net Sales for Product in the Territory are equal to or exceed [\*\*] Euros ([\*\*]€).

The [\*\*] percent royalty payment by AMT shall begin at the end of the first calendar year of sales in any country of the Territory. For the sake of clarity, the “end of the first calendar year” means December 31 of the first year of sales in any country of the Territory and does not mean the end of the first consecutive twelve months of sales in a country of the Territory.

3.2 All royalties payable under this Agreement in respect of a calendar year shall be due and payable in EURO within [\*\*] days after December 31 in that calendar year.

3.3 AMT shall pay the royalties annually in EURO. Payment of the royalties and other sums described in Section 3.1 shall be made by wire transfer to AVENTIS’s bank account as will be indicated in writing to AMT from time to time provided that AVENTIS has indicated its bank account details to AMT prior to the royalties or any other sums under this Agreement becoming due and payable. For the avoidance of doubt, the signature fee under Section 3.1(a) shall be due and payable following signature of the License Agreement by both parties and within [\*\*] days after receipt of an invoice setting out AVENTIS’s bank account details.

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3.4 If AMT fails to make a timely payment, interest shall accrue at a rate of [\*\*] % per month independent of sending a reminder to AMT.

3.5 AMT shall keep, and cause its Affiliates to keep, complete and correct records concerning the calculation of Net Sales and the royalties thereon. AMT shall be obliged to keep such records for [\*\*] years after the making of a royalty payment. For any calendar year during the term hereof and within [\*\*] days of the end of such calendar year, AMT shall send to AVENTIS a written report describing (i) the Net Sales of Product booked by AMT or its Affiliates by country and (ii) corresponding calculation of the royalties. In those cases where the amount due is calculated based on a currency other than the EURO, the amount due in EURO shall be calculated using the average rate of exchange for such currency, as quoted in the Financial Times, on the last business day of each of the calendar year to which such payment pertains.

3.6 AVENTIS shall have the right to have an independent accountant reasonably acceptable to AMT inspect AMT’s records and AMT Affiliates’ records of Net Sales and royalties. Such accountant shall have access, during ordinary business hours, to such records of AMT and/or its Affiliates in order to verify the accuracy of royalty payments made or payable hereunder. AVENTIS shall only have access to the report of the accountant as to whether or not the payments were accurate or inaccurate and to what extent, the accountant keeping all data and information of AMT confidential. The costs and expenses of the accountant’s inspections shall be borne by AVENTIS except in the event of an underpayment of greater than [\*\*]% of royalty payments payable hereunder, where the costs and expenses of the accountant’s inspections shall be borne by AMT.

## 4. REPRESENTATIONS AND WARRANTIES

4.1 REPRESENTATIONS AND WARRANTIES OF AVENTIS: AVENTIS represents and warrants that as at the Effective Date it is duly organized, validly existing and in good standing under the laws of France, that it has full corporate power and authority to enter into this Agreement and to carry out its provisions and more specifically the execution, delivery and performance by AVENTIS of this Agreement and its compliance with the terms and provisions hereof does not conflict with, or result in a breach of any of the

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terms and provisions of, or constitute a default under any agreement to which AVENTIS is a party. AVENTIS further represents and warrants that it is duly authorized to execute and deliver this Agreement and to perform its obligations hereunder and that the execution, delivery and performance of this Agreement by it does not require the consent, approval or authorization of or notice, filing or registration with any governmental or regulatory agency.

- 4.2 **REPRESENTATIONS AND WARRANTIES OF AMT:** AMT represents and warrants that as at the Effective Date it is duly organized, validly existing and in good standing under the laws of the Netherlands, that it has full corporate power and authority to enter into this Agreement and to carry out its provisions and more specifically the execution, delivery and performance by AMT of this Agreement and its compliance with the terms and provisions hereof does not conflict with, or result in a breach of any of the terms and provisions of, or constitute a default under any agreement to which AMT is a party. AMT further represents and warrants that it is duly authorized to execute and deliver this Agreement and to perform its obligations hereunder, and that the execution, delivery and performance of this Agreement by it does not require the consent, approval or authorization of or notice, filing or registration with any governmental or regulatory agency.

## 5. **PATENT MAINTENANCE AND DEFENSE**

- 5.1 AVENTIS shall maintain the Patents in the Territory and shall bear all expenses in connection with the foregoing during the term of this License Agreement.
- 5.2 If AVENTIS declines to maintain any Patents in any country in the Territory, then AVENTIS shall provide AMT with written notice thereof, and AMT shall have the right, subject, where applicable, to the preemptive right of UBC under any local patent law in the Territory, to have AVENTIS assign its share in such Patents to AMT free of charge and in a form approved by a patent attorney mutually agreed between the parties in the applicable country in the Territory ("Agreed Form"). AVENTIS shall give such notice at least [\*\*] days prior to the expiration of any official substantive deadline relating to the maintenance of the Patents in question. In any such circumstances AMT shall have the

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right to decide that AMT should continue to maintain such Patents and in such case AMT shall give written notice to AVENTIS together with the Agreed Form. AVENTIS shall upon receipt of any such notice from AMT transfer to AMT all its records and files relating to the relevant Patents and execute any documents in the Agreed Form to assign the Patents to AMT or otherwise transfer control of such maintenance to AMT and thereafter AMT shall be responsible for the cost and expense of maintaining such Patents. Notwithstanding the operation of this Section 5.2, AMT's payment obligations contained in Section 3.1 shall remain unaffected.

- 5.3 In the event a third party, through the actual or proposed manufacture, import, use, sale or offer for sale of a product competitive with the Product, infringes or is reasonably likely to infringe any Patent, AMT shall have the right, at its sole cost and expense, to institute, prosecute and control any action or proceeding with respect to such infringement. AVENTIS has the right to participate and be represented in such action by counsel of its own selection at its own expense, and also agrees to be joined as a party plaintiff, if necessary in any such action, and to give AMT reasonable assistance and needed authority to control, file and to prosecute such action.

## 6. **INDEMNIFICATION**

- 6.1 AVENTIS and/or its Affiliates shall indemnify and hold AMT and/or its Affiliates harmless from and against any and all claims, judgements, costs, awards, expenses (including, without limitation, reasonable attorney's fees) attributable or alleged to be attributable to the breach by AVENTIS of its representations and warranties under this License Agreement.
- 6.2 AMT and/or its Affiliates shall indemnify and hold AVENTIS and/or its Affiliates harmless from and against any and all claims, judgements, costs, awards, expenses (including, without limitation, reasonable attorney's fees) or liability of any kind attributable or alleged to be attributable to (i) the manufacture and/or use of the Product or marketing, sale and/or distribution of the Product by AMT and/or its Affiliates in the Territory or (ii) the breach by AMT of its representations and warranties under this License Agreement.

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## 7. **CONFIDENTIALITY**

- 7.1 Each party undertakes to keep strictly confidential the substance contained in this License Agreement and any and all information received from the other party in connection with this License Agreement (collectively referred to hereinafter as "Confidential Information") and to use it only for the purpose of this Agreement.
- 7.2 The receiving party undertakes to make the Confidential Information available only to those of its employees or to those employees of its Affiliates who in advance have been committed to confidentiality under conditions at least as stringent as contained herein.
- 7.3 The obligations to maintain confidentiality and to respect the restriction of the use shall not apply where, as properly evidenced by documentation:
- (a) the Confidential Information was previously known to the receiving party at the time of disclosure from sources other than the disclosing party and other than under an obligation of confidentiality; or
  - (b) the Confidential Information was generally available to the public or otherwise part of the public domain at the time of its disclosure; or
  - (c) the Confidential Information became generally available to the public or otherwise part of the public domain after its disclosure to the receiving party other than through any act or omission of the receiving party in breach of this License Agreement; or
  - (d) the Confidential Information is acquired in good faith in the future by the receiving party from a third party who has a lawful right to disclose such information and who is not under an obligation of confidence to the disclosing party with respect to such information; or

- (e) the Confidential Information is subsequently developed by or on behalf of the receiving party without use of the disclosing party's Confidential Information, as can be documented by reasonable proof.

7.4 The receiving party shall be entitled to disclose Confidential Information or parts thereof to authorities and/or courts to the extent required by law or order.

7.5 The obligations to maintain confidentiality shall survive the termination of this Agreement for [\*\*] years.

## 8. **TERM**

This License Agreement shall become effective on the date on which this Agreement is signed by the last party to sign and, unless terminated early pursuant to Section 9 hereof, shall continue to be in full force and effect until the expiration of the last-to-expire Patent in the Territory.

## 9. **TERMINATION**

9.1 Either party shall have the right to terminate this License Agreement immediately by written notice in the event that the other party shall default in any of the substantial obligations hereunder and fails to cure such default, if possible of cure, within [\*\*] days after receipt of a written notice from the party not being in default. For the purpose of this provision, substantial obligations shall include, without limitation, the payment by AMT of the amounts mentioned in Article 3 hereto.

9.2 AVENTIS shall have the right to terminate this License Agreement upon sixty (60) day prior written notice to AMT (i) in relation to France, Germany and the Netherlands in the event that AMT fails to launch the Product in France, Germany and the Netherlands within 4 years following the date on which this Agreement is signed by the last party to sign, (ii) on a country-by-country basis in the event that AMT fails to launch the Product in the remaining countries in the Territory (other than France, Germany and the Netherlands) 4 years following the date of launch of the Product in the last country of either France, Germany or the Netherlands and (iii) if there is a proceeding commenced against AMT under any bankruptcy act or under any present or future law for relief of debtors, or an action or proceeding is commenced to dissolve AMT, or AMT makes an assignment for the benefit of creditors or ceases to carry on business for any reason.

## 10. **EFFECTS UPON EXPIRATION OR TERMINATION**

Expiration or termination shall not affect obligations which have accrued earlier.

## 11. **NOTICES**

Any notices required or provided for use in this License Agreement shall be in writing and shall be given by facsimile or by certified mail prepaid and properly addressed to the address of the party to be served as shown below.

If to AVENTIS:                   Aventis Pharma S. A.  
20 avenue Raymond Aron  
92160 Antony  
France

Attn: President Directeur General  
Fax: +33.1.53.77.42.03

With a copy to Sanofi-Aventis, attn General Counsel, 174 avenue de France, 75013 Paris, France. Facsimile: +33.1.53.77.46.43.

If to AMT:                       Amsterdam Molecular Therapeutics B. V.  
Meibergdreef 61  
1105 BA Amsterdam  
The Netherlands

Attn: Prof. Sander J.van Deventer, MD, PhD, Chief Scientific Officer  
Fax:+31 (0) 20 566 9272

## 12. **FORCE MAJEURE**

Neither party hereto shall be liable to the other party for any losses or damages attributable to a default in or breach of this License Agreement which is the result of any cause beyond the reasonable control of such party and in this case the performance of obligations hereunder shall be suspended during, but no longer than the existence of such cause. The party affected by an event of force majeure shall inform the other party hereof without delay. Furthermore, it shall endeavour to take up its performance under this Agreement again as soon as possible.

## 13. **MISCELLANEOUS**

13.1 The relationship of the parties of this License Agreement is that of independent contractors. Neither party shall be the agent of the other and neither party is authorized to take any action binding on the other.



and

**UNIQUE BIOPHARMA B.V.**

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**AMENDMENT N°1 TO THE  
LICENSE AGREEMENT**

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**AMENDMENT N°1 TO THE LICENSE AGREEMENT** (the “Amendment”), with the effective date of June 28, 2013 (“Effective Date”),

**BY AND BETWEEN:**

**AVENTIS PHARMA S.A.**, a company incorporated under the laws of the France, with offices at 20 Avenue Raymond Aron, 92160 Antony, France (“Aventis”);

and

**uniQure biopharma B.V.** (formerly: Amsterdam Molecular Therapeutics (AMT) B.V.), a company incorporated under the laws of the Netherlands, with offices at Meibergdreef 61, 1105 BA Amsterdam, The Netherlands (“uniQure”),

each, a “Party” and together the “Parties”,

**BACKGROUND:**

- (A) The Parties have signed a License Agreement with an effective date of December 20, 2006 (hereinafter the “Agreement”);
- (B) The Parties desire that the Agreement be amended as set forth below in order to:
  - I. Change the name of uniQure from Amsterdam Molecular Therapeutics B.V. to uniQure biopharma B.V. This name change is the result of a transaction that took place on 30 March 2012, whereby Amsterdam Molecular Therapeutics (AMT) Holding N.V., a public company, was liquidated and all its operations and stocks were transferred to UniQure B.V., a privately held company;
  - II. Add language related to sublicensing, so that the possibility is created for uniQure to enter into sublicensing agreements under the patents licensed through the Agreement;
  - III. Modify the financial terms associated with royalty payments;
  - IV. Create reporting obligations regarding pricing and reimbursement of uniQure’s gene therapy product Glybera® and its development milestones, and
  - V. Add a Right of First Negotiation for Aventis.

**IT IS NOW AGREED AS FOLLOWS:**

**1. Modifications**

- I. In the Agreement, all references to “Amsterdam Molecular Therapeutics B.V.” are changed to “uniQure biopharma B.V”.
- II. In the Agreement, all references to “AMT” are changed to “uniQure”.
- III. Section 1.1 is deleted and replaced in its entirety with the following:

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1.1 “Affiliate” shall mean any company which Controls, is Controlled by or is under common Control with AVENTIS, and shall mean, with respect to uniQure, any company which Controls, is Controlled by or is under common Control with uniQure and shall mean, with respect to the Commercialization Partner, any company which Controls, is Controlled by or is under common Control with the Commercialization Partner. “Control” shall mean the direct and/or indirect legal or beneficial ownership of fifty percent (50%) or more of the capital stock or the voting rights of such company.

- IV. Section 1.5 is deleted and replaced in its entirety with the following:

1.5 “Net Sales” shall mean the gross sales invoiced for the Product by uniQure and its Affiliates, or by the Commercialization Partner and its Affiliates, as applicable, to third parties in the Territory less: (i) quantity and/or cash discounts, allowances actually allowed or given, and transportation and insurance costs for shipments to customers; (ii) credits or refunds actually allowed for returned Product; (iii) compulsory payments and rebates, accrued, paid or deducted pursuant to agreements (including but not limited to managed care agreements) or governmental regulations and (iv) sales and other excise taxes directly related to that sale, to the extent that such items are included in the gross invoice price (but not including taxes assessed against the income derived from such sale).

- V. Section 1.7 is deleted and replaced in its entirety with the following:

1.7 “Product” shall mean a gene therapy product containing a recombinant virus with a nucleic acid coding for a lipoprotein lipase (LPL) developed, used and/or commercialized by or for uniQure, the Commercialization Partner or their Affiliates where the activity in

relation to such product occurs in one of the countries of the Territory and is covered in whole or in part by a Valid Claim within the Patents in that country.

VI. A new Section 1.10 is added to the Agreement that reads as follows:

1.10 “Commercialization Partner” means uniQure’s partner for the commercialization of the Product.

VII. A new Section 1.11 is added to the Agreement that reads as follows:

1.11 “Fully Loaded Cost of Goods” shall mean the fully loaded cost of goods of the Product as defined in Annex 2.

VIII. A new Section 1.12 is added to the Agreement that reads as follows:

1.12 “Invoice Price” means the price invoiced by uniQure to the Commercialization Partner for the Products minus the Fully Loaded Cost of Goods.

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IX. New Sections 2.4 and 2.5 are added to the Agreement which read as follows:

2.4 uniQure shall have the right to grant sublicenses under the Patents to third parties for the purpose of developing, using, marketing, selling, offering for sale, manufacturing, distributing or otherwise commercializing Products, and having such activities performed, without the written consent of Aventis, on terms that permit uniQure to comply with its obligations set out in the Agreement.

2.5 Except for those expressly set forth in this Agreement, no other rights, licenses or sublicense rights are granted by Aventis or its Affiliates to uniQure.

X. Section 3.1 is deleted and replaced in its entirety with the following:

3.1 In consideration of the rights granted by Aventis to uniQure under this License Agreement, uniQure undertakes to effect the following payments:

(a) Ten thousand Euros (10,000 €) upon signature of the Agreement;

(b) Fifty thousand Euros (50,000 €) upon [\*\*];

(c) Seventy-five thousand Euros (75,000 €) at the [\*\*];

(d) Royalty payments the higher of:

Either

i (x) [\*\*] percent ([\*\*]%) of UniQure’s Net Sales of Product in the Territory or (y) if Product is being sold by Commercialization Partner and not uniQure, then [\*\*]percent ([\*\*]%) of the royalties (or other revenue) uniQure receives from its Commercialization Partner for its Net Sales of Product in the Territory plus [\*\*] percent ([\*\*]%) of the Invoice Price of Product in the Territory, in each case (x) or (y), as long as total cumulative Net Sales of Product are below [\*\*] Euros ([\*\*] €), and

ii (x) [\*\*] percent ([\*\*]%) of UniQure’s Net Sales of Product in the Territory or (y) if Product is being sold by Commercialization Partner and not uniQure, then [\*\*] percent ([\*\*]%) of the royalties (or other revenue) uniQure receives from its Commercialization Partner for its Net Sales of Product in the Territory plus [\*\*] percent ([\*\*]%) of the Invoice Price of Product in the Territory, in each case (x) or (y), as long as total cumulative Net Sales of Product are equal to or exceed [\*\*] Euros ([\*\*] €),

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or

iii [\*\*] percent ([\*\*]%) of Net Sales by Commercialization Partner of Product in the Territory.

The royalty payments by uniQure to Aventis shall begin at the end of the first calendar year of sales in any country of the Territory. For the sake of clarity, the “end of the first calendar year” means December 31 of the first year of sales in any country of the Territory and does not mean the end of the first consecutive twelve months of sales in a country of the Territory.

XI. A new Section 3.7 is added to the Agreement that reads as follows:

3.7 For a period of [\*\*] years from [\*\*], uniQure shall provide Aventis with written quarterly reports describing (i) the pricing and reimbursement status of Glybera®, including but not limited to, detailed minutes, transcripts or summaries (as available) of any communication, feedback, guidance or decisions from any reimbursement authority regarding Glybera®, as well as copies of any currently existing or future (during the [\*\*] years) reimbursement reports or advice received from any external consultants and (ii) uniQure’s pipeline development milestones. For the sake of clarity, this means that Aventis shall receive a total number of eight of such reports on or before the following dates: [\*\*].

XII. A new Section 3.8 is added to the Agreement that reads as follows:

- 3.8 uniQure is developing its proprietary construct AMT-021 for the treatment of Acute Intermittent Porphyria (AIP) in humans (the “AIP Product”). If after completion of phase I/II clinical trials regarding the AIP Product or for a period of [\*\*]months thereafter, uniQure contemplates entering into a partnership for the co-development and commercialization of the AIP Product, uniQure shall inform Aventis accordingly and offer Aventis a right of first negotiation to enter into a partnership agreement for such development and commercialization (the “Right of First Negotiation” or “ROFN”). uniQure shall provide Aventis with a summary of the existing clinical trial data regarding the AIP Product but any such summary shall not include any manufacturing information for the AIP Product unless specifically requested in writing by Aventis; in the event uniQure discloses manufacturing information to Aventis without its request, any such manufacturing information disclosed by or on behalf of uniQure shall not be treated as Confidential Information under the Agreement. Within [\*\*] business days after having received such summary from uniQure, Aventis shall decide whether it wishes to exercise its ROFN. If Aventis remains silent after such period or informs uniQure it does not wish to enter into such negotiation, uniQure shall be free to further develop and commercialize the AIP Product as it deems fit and enter into a partnership agreement with any third party without any obligation to Aventis. If

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Aventis wishes to exercise its ROFN, Aventis will notify uniQure within the [\*\*] day evaluation period and will then have [\*\*] months from such notification date to negotiate a term sheet. Upon mutual agreement of the term sheet, the Parties shall then enter into good faith discussions, meeting and negotiating at reasonable times with willingness to reach agreement regarding the development and commercialization of the AIP Product using commercially reasonable terms. Such negotiations shall be consistent with the aim of achieving a final definitive agreement within [\*\*] months from the start of said negotiations, and shall include, but not be limited to, responding promptly to proposals from the other Party and attending meetings at reasonable notice. If the Parties are unable to finalize the terms of a definitive agreement, uniQure shall be free to enter into an agreement with a third party. Aventis’s ROFN shall expire upon expiry of the Agreement pursuant to Section 8 or upon termination of this Agreement by uniQure pursuant to Section 9.1.

**2. Miscellaneous:** All the other provisions of the Agreement remain unchanged and in full force and effect between the Parties, and the terms and definitions used in the Agreement shall apply to this Amendment. To the extent any terms or provisions of this Amendment conflict with the terms and provisions of the Agreement, the terms and provisions of this Amendment shall prevail. This Amendment is made a part of the Agreement.

IN WITNESS WHEREOF, the Parties have caused this Amendment to be executed as of the Effective Date.

**Aventis Pharma S.A.**

By: /s/ Philippe Goupit  
 Name: Philippe Goupit  
 Title: Vice President Business Development  
 Date: 28/06/2013

**UniQure biopharma B.V.**

By: /s/ PJ Morgan  
 Name: PJ Morgan  
 Title: CFO  
 Date: 28 June 2013

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**Annex 2**

**Definition  
Fully Loaded Cost of Goods**

<b>Item per 50 L batch</b>	<b>Costs* [EUR]</b>
Clean room occupancy	[**]
Cell bank vial	[**]
Virus banks vials	[**]
Raw materials	[**]
External release assays (QC)	[**]
External QP	[**]
Personnel (MF, QC, QA)	[**]
Packaging (incl. release)	[**]
Stability study batch allocation	[**]
<b>Total</b>	<b>[**]</b>

Norms:

Number of patients per batch [\*\*]  
 Batch success rate [\*\*]%

**Result Fully Loaded Costs:**

**COG per patient** EUR [\*\*]  
**COG per batch** EUR [\*\*]

**COG relevant for Glybera Manufacturing Cost Reimbursement:**

**COG per patient** EUR [\*\*]  
**COG per batch** EUR [\*\*]

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Confidential Materials omitted and filed separately with the Securities and Exchange Commission. Double asterisks denote omissions.

### **NON-EXCLUSIVE LICENSE AGREEMENT**

This License Agreement (the “**Agreement**”), effective as of September 3, 2010 (the “**Effective Date**”), is by and between AskLêpios Biopharmaceutical, Inc., an entity organized and existing under the laws of the State of North Carolina, with its registered office located at 45 N. Chatham Parkway, Chapel Hill, NC 27517 (the “**AskBio**”), and Amsterdam Molecular Therapeutics (AMI) B.V., with offices located at Meibergdreef 61, 1100 DA Amsterdam, The Netherlands (“**AMT**”). AMT and AskBio may be hereinafter referred to individually as “**Party**” and jointly as “**Parties**”.

### **RECITALS**

WHEREAS, AskBio is the exclusive licensee of the **AskBio Patent Rights** (as defined below) pursuant to the amended and restated license agreement made and entered into on June 1, 2009 between The University of North Carolina at Chapel Hill and AskBio;

WHEREAS, AskBio has the right to grant sublicenses under such AskBio Patent Rights; and

WHEREAS, AMT desires to obtain a non-exclusive license under the AskBio Patent Rights upon the terms and conditions set forth below.

NOW, THEREFORE, for and in consideration of the mutual covenants contained herein and of other good and valuable consideration, the receipt and sufficiency of which are hereby acknowledged, AskBio and AMT hereby agree as follows:

### **AGREEMENT**

#### **1. Definitions.**

- a. “**AskBio Patent Rights**” means the patents and patent applications listed in Exhibit A hereto, including all provisionals, divisions, continuations, continuations-in-part, reissues, reexaminations, extensions, supplementary protection certificates, and foreign counterparts of any of the foregoing.
- b. “**Field**” means the treatment of lipoprotein lipase deficiency in humans.
- c. “**Product**” means GLYBERA® and/or any other pharmaceutical product which contains or consists of an AAV vector having an AAV genetic construct encoding LPL gene variant and which, in the absence of a license, would infringe any Valid Claim in the applicable country.
- d. “**Territory**” means worldwide.
- e. “**Valid Claim**” means any claim from an issued and unexpired AskBio Patent Right which has not been rejected, revoked or held unenforceable or invalid by a final, nonappealable decision of a court or other governmental authority of competent jurisdiction or unappealed within the time allowable for appeal, and which has not been explicitly disclaimed, or admitted to be invalid or unenforceable by AskBio through reissue, disclaimer or otherwise.

- 2. **Grant.** AskBio grants AMT a non-exclusive license (the “**License**”) to research, develop, make, have made, use, sell, offer for sale, have sold and import Products in the Field in the Territory. AMT shall have the right to grant sublicenses to third parties, without the written consent of AskBio, under the AskBio Patent Rights, on terms permitting AMT to comply with its obligations set out in this Agreement. AskBio does not grant AMT any other rights and all rights not granted to AMT hereunder are expressly reserved to AskBio. The License granted hereunder does not constitute a transfer or sale of any ownership rights in the AskBio Patent Rights.

AskBio — AMT  
Non-Exclusive License Agreement  
September 30, 2010

- 3. **Payment.** In consideration of the rights granted hereunder, and subject to the terms and conditions of this Agreement, AMT shall pay to AskBio the following nonrefundable license fees:

- a. Signature Fee: US\$50,000 upon the Effective Date of this Agreement; and
- b. License Maintenance Fee: US\$[\*\*] due within [\*\*] days of each anniversary of the Effective Date of this Agreement.

- 4. **Warranties by AskBio and Disclaimer.** AskBio hereby represents and warrants to AMT that (i) to AskBio’s best knowledge, the University of North Carolina at Chapel Hill is the sole owner of the AskBio Patent Rights and at the Effective Date the license is in force; (ii) AskBio has obtained from the University of North Carolina at Chapel Hill the exclusive, world-wide, rights for all applications under the AskBio Patents Rights; (iii) AskBio is entitled to grant the License granted hereunder; (iv) AskBio has not granted any license to any third party that would be inconsistent with the license granted to AMT hereunder; and (v) to AskBio’s best knowledge, the exercise of the AskBio Patent Rights does not infringe any other intellectual property right of AskBio or any intellectual property right of any third party. EXCEPT AS SET FORTH IN THIS SECTION 4, ASKBIO DISCLAIMS ANY AND ALL REPRESENTATION AND WARRANTIES, EXPRESS OR IMPLIED, INCLUDING WITHOUT LIMITATION ANY WARRANTY OF NON-INFRINGEMENT, TITLE, MERCHANTABILITY, OR FITNESS FOR A PARTICULAR PURPOSE. THE ASKBIO PATENT RIGHTS ARE PROVIDED TO AMT “AS-IS”, WITH ANY ACCOMPANY SERVICES, REPRESENTATIONS, OR WARRANTIES FROM ASKBIO.

- 5. **Limitation of Liability.** AMT assumes all liability for damages that may arise from the practice of the AskBio Patent Rights by AMT. AskBio will not be liable to AMT for any loss, claim, or demand made by AMT, or made against AMT by any third party, due to or arising from the practice of the AskBio Patent Rights by AMT, except to the extent permitted by law when caused by the gross negligence or willful misconduct of AskBio or breach of warranties by AskBio as set forth in Article 4 hereof. IN NO EVENT SHALL AMT BE ENTITLED TO RECOVER FROM ASKBIO ANY

SPECIAL, INDIRECT, INCIDENTAL, CONSEQUENTIAL, OR PUNITIVE DAMAGES, INCLUDING BUT NOT LIMITED TO LOST PROFITS, ARISING OUT OF OR IN ANY WAY RELATED TO THIS AGREEMENT OR THE PRACTICE OF THE ASKBIO PATENT RIGHTS BY AMT, ITS AFFILIATES, PARENT COMPANY, OR JOINT VENTURERS, REGARDLESS OF THE NATURE OF THE CLAIM, WHETHER IN CONTRACT, TORT, INTELLECTUAL PROPERTY, INFRINGEMENT, OR OTHER ACTION, AND WHETHER AMT HAS INFORMED THE ASKBIO OF ANY POSSIBLE DAMAGES.

6. **Indemnification.** AMT agrees to indemnify, defend, and hold harmless AskBio, its officers, agents, and employees from and against any and all claims, liabilities, demands, damages, losses, costs and expenses (including costs and reasonable attorney's fees) or claims for injury or damages arising out of or related to AMT's performance hereunder, including those that are caused by or result from AMT's practice of the AskBio Patent Rights, save for claims, liability, demands, damages, losses, costs and expenses that result from the gross negligence or willful misconduct of AskBio or from breach by AskBio of its warranties set forth in Article 4.

## 7. Term and Termination.

7.1. **Term.** The term of this Agreement shall begin on the Effective Date and shall, on a country-by-country basis, end on the earlier of June 5, 2016 or the last date on which Products are covered by a Valid Claim in such country.

7.2. **Early Termination by AMT.** AMT may terminate this Agreement at any time upon thirty (30) days notice to AskBio.

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7.3. **Early Termination by AskBio.** AskBio may terminate this Agreement (i) upon a material breach that is not cured within ten (10) days of written notice thereof, or (ii) if AMT or any of its sublicensees challenges, or assists others challenging, the validity, patentability, enforceability and/or non-infringement of any of the AskBio Patent Rights or otherwise opposes any of the AskBio Patent Rights.

7.4. **Effect of Early Termination.** Upon early termination of this Agreement, the license granted to AMT hereunder shall immediately terminate, and AMT shall immediately cease all use of the AskBio Patent Rights.

## 8. Miscellaneous.

8.1. **Relationship of the Parties.** For the purpose of this Agreement and all services to be provided hereunder, both parties shall be, and shall be deemed to be, independent contractors and not agents or employees of the other. Neither party shall have authority to make any statements, representations or commitments of any kind, or to take any action, that will be binding on the other party.

8.2. **Notices.** Any notice or other communication required or permitted to be given to either Party hereto shall be deemed to have been properly given provided it is delivered to the Party at the respective address set forth below, or as otherwise designated by prior written notice provided to the other Party. Any such notice or communication shall be deemed to be effective on the date of delivery if delivered in person, or on the date of mailing if delivered by certified mail.

If to AskBio: Asklêpios Biopharmaceutical, Inc.  
45 N. Chatham Parkway  
Chapel Hill, NC 27517  
Attention: Jade Samulski

If to AMT: Amsterdam Molecular Therapeutics B.V.  
Meibergdreef 61  
1100 DA Amsterdam, The Netherlands  
Attention: Piers Morgan

8.3. **Publicity; Advertising.** Neither of the Parties will use the other Party's name or the name of any member of that Party's personnel in any publicity (including press releases) relating to this Agreement or in any advertising, packaging or other promotional material, without the prior written approval of the other Party, except as may be required by law, regulation or legal process.

8.4. **No Modification/Amendments.** This Agreement, including the exhibits, may be changed only by written agreement of the Parties and in accordance with the terms of this Agreement; no amendment of any provision of this Agreement shall be valid unless the same shall be in writing and signed by the Parties.

8.5. **No Waiver.** No waiver by a Party of any right or remedy hereunder shall be valid unless the same shall be in writing and signed by the Party giving such waiver. No waiver by a Party with respect to any default, misrepresentation, or breach of warranty or covenant hereunder shall be deemed to extend to any prior or subsequent default, misrepresentation, or breach of warranty or covenant hereunder or affect in any way any rights arising by virtue of any prior or subsequent such occurrence.

8.6. **Severability.** Any term or provision of this Agreement that is invalid or unenforceable in any situation in any jurisdiction shall not affect the validity or enforceability of the remaining terms and provisions hereof or the validity or enforceability of the offending term or provision in any other situation or in any other jurisdiction. If the final judgment of a court of competent jurisdiction declares that any term or provision hereof is invalid or unenforceable, the Parties agree that the court making the determination of invalidity or

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unenforceability shall have the power to limit the term or provision, to delete specific words or phrases, or to replace any invalid or unenforceable term or provision with a term or provision that is valid and enforceable and that comes closest to expressing the intention of the invalid or unenforceable term or provision, and this Agreement shall be enforceable as so modified.

- 8.7. Assignment. This Agreement, and the rights and obligations hereunder, may not be assigned or transferred, in whole or in part, by either Party and AskBio shall not assign its rights under the license agreement with University of North Carolina at Chapel Hill to any third party without the prior written consent of the other Party, except that each Party may assign this Agreement without the consent of the other Party in connection with the merger, consolidation or sale of all or substantially all of its assets, stock or business to which this Agreement relates.
- 8.8. Applicable Law. This Agreement shall be governed by the laws of the state of New York, United States, without regard to conflicts of laws principles.
- 8.9. Counterparts. This Agreement may be executed in two or more counterparts, each of which shall be deemed an original but all of which together shall constitute one and the same instrument.
- 8.10. Entire Agreement. This Agreement, including all attachments hereto, constitutes the entire agreement of the Parties with regard to its subject matter, and supersedes all previous written or oral representations, agreements and understandings between the Parties.

IN WITNESS WHEREOF, AskBio and AMT have executed this Agreement by their respective officers hereunto duly authorized, on the day and year hereinafter written.

<b>For Asklēpios Biopharmaceutical, Inc.</b>		<b>For Amsterdam Molecular Therapeutics (AMT) B.V.</b>	
By:	<u>/s/ Jude Samulski</u>	By:	<u>/s/ P J Morgan</u>
Name:	<u>Jude Samulski</u>	Name:	<u>P J Morgan</u>
Title:	<u>President</u>	Title:	<u>CFO</u>

**Exhibit A**  
**AskBio Patent Rights**

Patent Filing	Publication Date	Filing Date	Status as of the Effective Date
【**】	【**】	【**】	【**】
【**】	【**】	【**】	【**】
【**】	【**】	【**】	【**】
【**】	【**】	【**】	【**】
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【**】	【**】	【**】	【**】

Confidential Materials omitted and filed separately with the  
Securities and Exchange Commission. Double asterisks denote  
omissions.

**LICENSE AGREEMENT BETWEEN**  
**SALK INSTITUTE FOR BIOLOGICAL STUDIES**  
**AND**  
**AMSTERDAM MOLECULAR THERAPEUTICS BV**  
**RNA EXPORT ELEMENT WPRE**  
**(Woodchuck Hepatitis Virus Posttranscriptional Regulatory Element)**  
**GENE THERAPY**

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## LICENSE AGREEMENT

Effective as of February 8, 2008 (“Effective Date”), THE SALK INSTITUTE FOR BIOLOGICAL STUDIES, a nonprofit public benefit corporation organized and existing under the laws of the State of California, U.S.A. (“Salk”), and Amsterdam Molecular Therapeutics BV, a corporation organized and existing under the laws of The Netherlands (“Licensee”), agree as follows:

### 1. DEFINITIONS

1.1 “**Licensed Patents**” shall mean the patents and patent applications listed on Schedule A hereto, and any divisions, continuations, continuations-in-part, reissues and renewals, if any, of the aforementioned.

1.2 “**Biological Materials**” shall mean the constructs identified in Schedule B (and any materials transferred during the Term) together with any progeny, mutants, modified derivatives, or unmodified derivatives thereof supplied by Salk or created by Licensee that do not significantly alter the original function(s) and/or activity of the construct. A “modified derivative” means any substance that Licensee may create using the constructs supplied hereunder, or created practicing the Licensed Patents, which substance constitutes a modified element derived from, but functionally equivalent to, the original construct (the WPRE element) identified in Schedule B. An “unmodified derivative” means any substance that Licensee may create using the constructs supplied hereunder, or created practicing the Licensed Patents, which substance constitutes an important unmodified functional subunit of the originally supplied material.

1.3 “**Licensed Technology**” shall mean the Licensed Patents and the Biological Materials.

1.4 “**Licensed Product**” shall mean any product which is composed of or incorporates or is directly or indirectly discovered, developed, produced and/or identified using the Licensed Technology. The term Licensed Product shall also include any product, the manufacture, use, importation, sale or offer for sale of which in the absence of this license would infringe the Licensed Patents.

1.5 “**Field of Use**” shall mean the right to use the Licensed Technology for Gene Therapy.

1.6 “**Net Sales**” shall mean the gross sales price actually charged in arm’s length sales by Licensee or its Affiliates to any third party, in the sale of a Licensed Product less:

- (i) shipping, storage, packing and insurance expenses, each as actually paid or allowed;
- (ii) distributor discounts;
- (iii) amounts repaid or credited by reason of rejections, defects or returns or because of retroactive price reductions; and

- (iv) sales and other excise taxes, use taxes, tariffs, export license fees, government charges and duties actually paid or allowed.

1.7 “**Affiliate**” shall mean any entity which is controlled by or is under common control with Licensee, where “control” means beneficial ownership of more than fifty percent (50%) of the voting shares or securities or the ability otherwise to elect a majority of the board of directors or other managing authority.

1.8 “**Gene Therapy**” shall mean the administration directly to a human of a gene transfer system comprising genetic material that encodes a moiety, wherein such moiety serves a material function in the treatment of or prevention of disease, the modification or manipulation of the expression of a gene product(s), or the alteration of the biological properties of living cells in humans. To clarify, Gene Therapy may include use in vitro in vertebrate cells or administration of a gene transfer system to non-human vertebrate animals for research and development only.

### 2. BACKGROUND

2.1 The inventions disclosed and claimed in the Licensed Patents were conceived and/or developed in the laboratory of [\*\*]. Licensed Patents may have utility, among other tilings, for gene therapy, drug discovery and development and protein expression in cells or transgenic animals.

2.2 Salk represents that it is the owner of and/or has the right to grant licenses under the Licensed Patents.

2.3 Salk and Licensee wish to enter into a license that will encourage the use of the Licensed Technology in the field of Gene Therapy.

### 3. GRANT OF LICENSE

3.1 **Licensed Technology.** Subject to the terms and conditions hereof, Salk grants to Licensee and its Affiliates a worldwide, non-exclusive, non-transferable license in and to the Licensed Technology to research, have researched, develop, have developed, make, have made, use, have used, import, have imported, offer for sale, sell and have sold Licensed Products solely in the Field of Use.

3.2 **Sublicenses.** Licensee shall not have the right to grant sublicenses. However, upon request by Licensee, Salk shall grant nonexclusive, worldwide licenses in and to the Patent Rights to for-profit Collaborators of Licensee on terms substantially the same as those set forth herein.

### 4. ROYALTIES AND FEES

4.1 As partial consideration for the rights granted to Licensee under this Agreement and as a non-refundable and non-creditable license fee, Licensee shall pay to Salk (a) Thirty Thousand Dollars (\$30,000.00) in cash, due upon execution, and (b) [\*\*] Dollars (\$[\*\*]) in cash

annually thereafter for the Term, beginning on the one-year anniversary date of the Effective Date. The annual fee is non-refundable and not subject to proration.

4.2 Licensee agrees to pay to Salk the sum of five thousand dollars (\$5,000) in cash, due upon execution, as payment of a share of the cost of general administration, filing, prosecution and maintenance of the Patent Rights.

4.3 Licensee shall pay to Salk the following royalties:

- (i) [\*\*] percent ([\*\*]%) on Net Sales of Licensed Products up to [\*\*] dollars (\$[\*\*]), calculated from the Effective Date,
- (ii) [\*\*] percent ([\*\*]%) on Net Sales of Licensed Products [\*\*] dollars (\$[\*\*]) up to [\*\*] dollars (\$[\*\*]), calculated from the Effective Date,
- (iii) [\*\*] percent ([\*\*]%) on Net Sales of Licensed Products over [\*\*] dollars (\$[\*\*]), calculated from the Effective Date.

4.4 All payments made to Salk under Paragraphs 4.1 and 4.2 of this Agreement shall be made on the dates defined therein. Royalty payments made to Salk under Paragraph 4.3 of this Agreement shall be made quarterly. All payments made to Salk under this Agreement shall be made by wire transfer to the account of Salk at:

Send wire to:	Bank of America, San Francisco, CA
Route No. (ABA#):	0260-0959-3
Swift No.:	BOFAUS3N
Credit to:	The Salk Institute for Biological Studies
Account No.:	[**]
Branch Name:	B of A San Diego Commercial Banking Office #1450
Additional Message:	Sender's name, purpose of wire, & Attn: Constance Mueller, Patent and License Administrator

or any other bank account that may be notified in writing by Salk to Licensee from time to time.

4.5 No royalty payment shall be altered by any tax on royalties which may be imposed on, applied to or withheld from the payment. Licensee shall be responsible for the satisfaction of the taxes so that the payments to Salk are not reduced thereby.

## 5. REPORTING AND ACCOUNTING

5.1 Licensee agrees to keep proper records of scientific research and keep books of account in accordance with international financial reporting standards. Such records and books shall include all information necessary for the accurate determination of royalty and other payments to Salk. Following launch of Licensed Product, Licensee agrees to deliver to Salk, within [\*\*] days after each calendar quarter, a report showing the information on which payments herein provided are calculated, including a breakdown of income from sales of each Licensed Product, and to accompany each such report with the payment shown to be due

thereby. All amounts accrued for the benefit of Salk shall be deemed held in trust for the benefit of Salk until payment of such amounts is made pursuant to this Agreement.

5.2 Licensee agrees to make a written report to Salk within [\*\*] days after the termination date of this Agreement, reporting all royalty income payable hereunder which was not previously reported to Salk.

5.3 Except as Salk may otherwise instruct Licensee under Paragraph 5.6, all amounts payable hereunder by Licensee to Salk shall be payable in United States currency in San Diego, California.

5.4 On reasonable written notice, Salk, at its own expense, shall have the right to have an independent party reasonably acceptable to the Licensee, inspect and audit the books and records of Licensee and its Affiliates at their offices during usual business hours for the sole purpose of, and only to the extent necessary for, determining the correctness of payments due under this Agreement. Such examination with respect to any fiscal year shall not take place later than [\*\*] years following the expiration or termination of this Agreement. The expense of any such audit shall be borne by Salk; provided, however, that, if the audit discloses an error in excess of [\*\*] percent ([\*\*]%) in favor of Licensee, then Licensee shall pay, in addition to the amount of any underpayment, the cost to Salk of the audit.

5.5 Royalties based on Net Sales in any foreign country shall be payable to Salk in the United States in United States Dollars. Dollar amounts shall be calculated using the foreign exchange rate, as published by the Wall Street Journal, in effect for such foreign currency on the last business day of each quarter for which a report is required. Where royalties are due for Net Sales in a country where, for reasons of currency, tax or other regulations, transfer of foreign currency out of such country is prohibited, Licensee has the right to place Salk's royalties in a bank account in such country in the name of and under the sole control of Salk; provided, however, that the bank selected be reasonably acceptable to Salk and that Licensee inform Salk of the location, account number, amount and currency of money deposited therein. After Salk has been so notified, those monies shall be considered as royalties duly paid to Salk and will be completely controlled by Salk.

5.6 Licensee shall be responsible for any and all taxes that may be levied by a proper taxing authority on royalties or other payments accruing to Salk under this Agreement. Such taxes may not be deducted from royalties or other payments to be paid to Salk hereunder. Licensee acknowledges that Salk, as a not-for-profit corporation, does not qualify under U.S. tax laws for a tax credit on any taxes paid by Licensee.

5.7 **Late Payments.** Late payments shall be subject to a charge of [\*\*] percent ([\*\*]%) per month compounded, or \$[\*\*] per month, whichever is greater. The payment of such late charges shall not prevent Salic from exercising any other rights it may have as a consequence of the lateness of any payment.

## 6. BIOLOGICAL MATERIALS

6.1 Upon execution of this Agreement, Salk or its designee shall make available to Licensee the Biological Materials described in Schedule B. Thereafter, Licensee may request

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and obtain from Salk additional quantities of said Biological Materials that Salk has in its possession and does not need for its own research or to meet other contractual obligations, at reasonable charge by Salk for handling and shipping. If Salk fails to fulfill the request of Licensee for Biological Materials, Licensee shall be free to produce such Biological Materials itself, or to purchase such Biological Materials from a source authorized by Salk.

6.2 Title to Biological Materials shall remain with Salk. Licensee shall have only the right to use Biological Materials in accordance with the grant of rights under Paragraph 3 hereof, and shall not use Biological Materials for any other purpose. Title to Licensed Products shall remain with the Licensee save that the Licensee acknowledges that it cannot make, have made, use, have used, import, have imported, offer for sale, sell or have sold Licensed Products without a license from Salk. Licensee shall not permit Biological Materials or any sample thereof to be distributed or delivered to any person whatsoever, other than to its own employees and those of its Affiliates to be used as permitted herein. Licensee shall refer to Salk requests for Biological Materials from other research investigators or others.

6.3 Licensee shall notify Salk of any improvements (including, but not limited to, modified derivatives and variants) to the Biological Materials made by Licensee or its Affiliates, irrespective of whether any such improvements were made in collaboration with Salk or any of its employees. At Salk's request, Licensee shall provide the laboratory of [\*\*] with reasonable quantities of such improvements, and scientists in the laboratory of [\*\*] shall have the right to use them for their internal research. Title to all such improvements shall remain with Licensee.

6.4 Licensee acknowledges that Biological Materials are experimental in nature and may have unknown characteristics. Licensee further agrees to use prudence and reasonable care in the use, handling, storage, transportation, disposition and containment of Biological Materials and all products derived therefrom.

6.5 Licensee shall comply with all applicable Governmental laws and regulations, and with all published Governmental guidelines, pertaining to the use, handling, storage, transportation, disposition and containment of Biological Materials and all products derived therefrom.

6.6 BIOLOGICAL MATERIALS ARE PROVIDED WITHOUT WARRANTY OF MERCHANTABILITY OR FITNESS FOR A PARTICULAR PURPOSE OR ANY OTHER WARRANTY, EXPRESS OR IMPLIED.

## 7. OWNERSHIP OF INTELLECTUAL PROPERTY

Licensee (for itself and its Affiliates) acknowledges and agrees that Salk is and shall remain (as to Licensee) the owner of the Licensed Patents and Biological Materials, subject to the rights of the Federal Government as set forth in 35 U.S.C. §200 et seq., and that Licensee (including its Affiliates) has no rights in or to the Licensed Patents and Biological Materials other than the rights specifically granted herein.

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## 8. MAINTENANCE OF PATENT RIGHTS

8.1 Salk shall have full control over prosecution and maintenance of the patents contained in the Patent Rights.

8.2 In the event Licensee's use of Licensed Technology becomes the subject of a claim for patent or other proprietary right infringement anywhere in the world, the parties shall promptly give notice to the other and meet to consider the claim and the appropriate course of action.

## 9. TERM AND TERMINATION

9.1 **Term.** Unless earlier terminated under this Paragraph 9, this Agreement shall become effective as of the date of this Agreement and expire on a country-by-country basis on the later to occur of the following (the "Term"):

- (a) fifteen (15) years from and after the Effective Date of this Agreement; or
- (b) the date of expiration of the last to expire of any patent included in the Licensed Patents; or
- (c) abandonment of the last remaining patent application included in the Licensed Patents.

9.2 **Termination by Either Party.** This Agreement may be terminated by either party, if the other party substantially fails to perform or otherwise materially breaches any of the material terms, covenants or provisions of this Agreement, such termination to be effected by giving written notice of intent to terminate to the breaching party stating the grounds therefor. The party receiving the notice shall have [\*\*] days thereafter to correct such breach. If such breach is not corrected within said [\*\*] days after notice as aforesaid, then this Agreement shall automatically terminate.

### 9.3 Consequences of Termination.

(a) In the event of expiration of this Agreement pursuant to Paragraph 9.1 Licensee and its Affiliates shall have a fully paid-up, royalty free license in and to the Licensed Technology to research, have researched, develop, have developed, make, have made, use, have used, import, have imported, offer for sale, sell and have sold Licensed Products in the Field of Use.

- (b) In the event of expiration of this Agreement or termination of the Agreement for any reason whatsoever:

(i) Licensee shall not thereby be discharged from any liability or obligation to Salk which became due or payable prior to the effective date of such expiration or termination; and

(ii) The rights and obligations of the parties under Paragraphs 6, 7, 9.3, 10, 11, 12, 13 and 14 shall survive any termination of this Agreement.

(iii) Licensee shall promptly return all materials, samples, documents, information, and other materials which embody or disclose Licensed Technology; provided, however, that Licensee shall not be obligated to provide Salk with proprietary information which Licensee can show that it independently developed. Licensee shall be permitted to retain copies of technical information in documentary form obtained from Salk where required to do so by reason of any statute, ordinance or regulation of any federal, state or local governmental entity.

(c) In the event of termination of the Agreement pursuant to Paragraph 9.2:

(i) Licensee agrees not to use Licensed Technology or said technical information after termination of this Agreement. If at termination Licensee or its Affiliates then possess Licensed Product, have started the manufacture thereof or have accepted orders therefor, Licensee and its Affiliates shall have the right to sell their inventories thereof, complete the manufacture thereof and market such fully manufactured Licensed Product, in order to fulfill such accepted orders, subject to the obligation of Licensee to pay Salk the royalty payments therefor as provided in Paragraph 4 of this Agreement;

(ii) Subject to Paragraph 9.3(c)(i), Licensee shall discontinue, and shall cause its Affiliates to discontinue, the manufacture, use, marketing and sale of Licensed Products;

(iii) Licensee shall provide a final report of the type described in Paragraph 5.1, including any allowable post-termination sales; and

(iv) All rights transferred by Salk to Licensee hereunder shall revert to Salk, and Licensee agrees to execute all instruments necessary and desirable to revest said rights in Salk.

## **10. AGENCY**

Licensee shall not be deemed to be an agent of Salk as a result of any transaction under or related to this Agreement, and shall not in any way pledge Salk's credit or incur any obligation on behalf of Salk.

## **11. USE OF SALK'S NAME**

Licensee may make it known in promotional and technical literature that Licensed Products are offered under license from Salk; provided, however, that such use shall not state or imply that Salk has any relationship with Licensee other than as licensor-licensee.

## **12. WARRANTY**

Subject to Salk's obligations to the U.S. Government under Public Law 96-517, as amended, and any implementing regulations, Salk warrants to the best of its knowledge and

belief that it is the owner of the Licensed Patents, free of any liens, encumbrances, restrictions and other legal and equitable claims.

## **13. DISCLAIMERS**

13.1 Nothing in this Agreement shall be construed as:

(a) a warranty or representation by Salk as to the validity or scope of the Licensed Patents;

(b) a warranty or representation by Salk that anything made, used, sold or otherwise disposed of under any license granted in this Agreement is or will be free from infringement of patents of third parties;

(c) an obligation of Salk to bring or prosecute actions or suits against third parties for infringement of Licensed Patents;

(d) conferring the right to use in advertising, publicity or otherwise any trademark, trade name, insignia, name, or names, or any contraction, abbreviation, adaptation of Salk, except to identify, where appropriate, Salk as the licensor of the Licensed Patents;

(e) an obligation of Salk to furnish any know-how; or

(f) a grant by implication, estoppel, or otherwise of any licenses under patent applications or patents of Salk or other persons other than as provided in Paragraph 3 hereof.

13.2 SALK MAKES NO REPRESENTATION, EXCEPT AS EXPRESSLY SET FORTH IN PARAGRAPH 12 OF THIS AGREEMENT, AND EXTENDS NO WARRANTY OF ANY KIND, EITHER EXPRESS OR IMPLIED, INCLUDING WARRANTIES AS TO TITLE, MERCHANTABILITY OR FITNESS FOR A PARTICULAR PURPOSE.

13.3 In no event will Salk be liable for any incidental, special or consequential damages resulting from the exercise of Licensee's rights under the license granted pursuant to this Agreement or the use of the Licensed Patents.



## **14. INDEMNIFICATION AND INSURANCE**

14.1 Licensee agrees to indemnify, hold harmless and defend Salk, its trustees, officers, employees and agents, the sponsors of the research that led to the Licensed Technology, and the inventors of the patents and patent applications included in the Licensed Patents against any and all liability and/or damages with respect to any claims, suits, demands, judgments or causes of action arising out of third party claims concerning (a) the research, development, manufacture, storage, sale or other distribution, or any other use of Licensed Products or Licensed Technology, or exercise of rights granted hereunder, by Licensee, its Affiliates, distributors, agents or representatives; (b) the use by end-users and other third parties of such Licensed Products; and/or (c) any representation, warranty or statement by Licensee or its Affiliates, distributors, agents or representatives, concerning Salk, the Licensed Technology or the Licensed Patents. In the event any such claims, demands or actions are made, Licensee shall

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defend Salk at Licensee's sole expense by counsel selected by Licensee, subject to approval by Salk, which is not to be unreasonably withheld. No settlement, consent judgment or other voluntary final disposition may be entered into without the prior written consent of Salk, which consent shall not be unreasonably withheld.

14.2 In addition to the foregoing, Licensee shall maintain, during the Term, comprehensive general liability insurance, including products liability insurance, with reputable and financially secure insurance carriers to cover the activities of Licensee and its Affiliates. Licensee shall maintain comprehensive general liability insurance beyond the expiration or termination of this Agreement during any period that any product, process, or service, relating to, or developed pursuant to, this Agreement is being commercially distributed or sold by Licensee, or an Affiliate or agent of Licensee; and thereafter for a period of [\*\*] years.

## **15. ASSIGNMENT**

This Agreement may not be assigned by Licensee without the prior written consent of Salk, which shall not unreasonably be withheld; provided, however, that Licensee may assign all of its rights and obligations under this Agreement in connection with a merger, sale of assets or other transaction involving a change in control of Licensee's business. Salk shall release Licensee of its rights and obligations under this agreement upon receipt of writing from successor or assignee expressly agreeing to be bound by all the terms and provisions of this Agreement. Upon assignment of this Agreement to a successor or assignee the term "Licensee" as used herein shall mean such successor assignee.

## **16. U.S. MANUFACTURE**

To comply with US Government regulations for the licensing of federally funded inventions, Licensee and its Affiliates will commit that Licensed Products sold in the US will be manufactured substantially in the US. Notwithstanding the foregoing, if during the term of the License Agreement Licensee or its Affiliates can provide compelling evidence to Salk that such manufacture in the US would impose an extraordinary burden on Licensee or its Affiliates, Salk shall at that time agree to seek a waiver from the US Government with respect to the requirement that Licensed Products for sale in the US be manufactured substantially in the US. Licensee understands that Salk cannot guarantee that such waiver can be obtained. Licensee shall bear all costs associated with seeking such waiver.

## **17. FOREIGN REGISTRATION**

Licensee agrees to register this Agreement with any foreign governmental agency that requires such registration, and Licensee shall pay all costs and legal fees in connection therewith. In addition, Licensee shall assure that all foreign laws affecting this Agreement or the sale of Licensed Products are fully satisfied.

## **18. EXPORT CONTROLS**

It is understood that Salk is subject to United States laws and regulations controlling the export of technical data, computer software, laboratory prototypes and other commodities (such laws include the Arms Export Control Act, as amended and Export Administration Act), and that

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its obligations hereunder are contingent on compliance with applicable United States export laws and regulations. The transfer of certain technical data and commodities by Licensee may require a license from the cognizant agency of the United States Government and/or written assurances by Licensee that Licensee shall not export data or commodities to certain foreign countries without prior approval of such agency. Salk neither represents that a license shall not be required nor that, if required, it shall be issued. Licensee shall not engage in any activity in connection with this Agreement that is in violation of any applicable U.S. law.

## **19. NOTICES**

19.1 All notices, demands or other writings provided to be given, made or sent, or which may be given, made or sent by either party to the other shall be deemed to have been fully given, made or sent when made in writing and deposited in the first-class mail, postage prepaid, or delivered to a commercial courier, charges prepaid, and addressed as follows:

To Salk: Salk Institute for Biological Studies  
10010 North Torrey Pines Road  
La Jolla, California 92037 U.S.A.  
Attn: Licensing Administrator  
Office of Technology Management

To Licensee: Amsterdam Molecular Therapeutics BV  
Meibergdreef 61  
PO Box 22506  
1100 DA Amsterdam  
The Netherlands

19.2 Either party may, upon written notice, change the address to which any notice or payment is made.

## **20. APPLICABLE LAW AND DISPUTE RESOLUTION**

20.1 This Agreement is made in accordance with and shall be governed and construed in accordance with the laws of the State of California, as applied to contracts executed and performed entirely within the State of California, without regard to conflict of laws rules.

20.2 The parties hereby irrevocably submit to the jurisdiction of a court of competent jurisdiction in the State of California, San Diego County, and, by execution and delivery of this Agreement, each (a) accepts, generally and unconditionally, the jurisdiction of such court and any related appellate court, and (b) irrevocably waives any objection it may now or hereafter have as to the venue of any such suit, action or proceeding brought in such court or that such court is an inconvenient forum.

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20.3 If a dispute arises between the parties relating to the interpretation or performance of this Agreement or the grounds for the termination thereof, the parties agree to hold a meeting, within [\*\*] days of a request by either party for such meeting, attended by individuals with decision-making authority regarding the dispute, to attempt in good faith to negotiate a resolution of the dispute prior to pursuing other available remedies. If the dispute remains unresolved [\*\*] days after the first meeting for the purpose of dispute resolution, then each party shall have the right to pursue alternate dispute resolution or other remedies legally available to resolve the dispute. In the case of such alternate dispute resolution or other legal remedies, the prevailing party will be entitled to receive from this other party its reasonable attorneys' fees and costs. If both parties receive judgment in any dollar amount, the court will determine the prevailing party, taking into consideration the merits of the claims asserted by each party, the amount of the judgment received by each party and the relative equities between the parties.

## **21. NONWAIVER**

The waiver of either party hereto of any right hereunder or of the failure to perform or of a breach by the other party shall not be deemed a waiver of any other right hereunder or of any other breach or failure by said other party whether of a similar nature or otherwise.

## **22. SEVERABILITY**

If any term, condition or provision of this Agreement is held to be unenforceable by a court having proper jurisdiction for any reason, it shall, if possible, be interpreted rather than voided, in order to achieve the intent of the parties to this Agreement to the extent possible. In any event, all other terms, conditions and provisions of this Agreement shall be deemed valid and enforceable to the full extent of the law.

## **23. HEADINGS**

The headings used in this Agreement are for convenience of reference only and are not intended to be a part of or to affect the meaning or interpretation of this Agreement.

## **24. AMENDMENT**

No amendment or modification hereof shall be valid or binding upon the parties unless made in writing and signed by both parties.

## **25. FORCE MAJEURE**

Any delays in performance by any party under this Agreement (other than the payment of monies due) shall not be considered a breach of this Agreement if and to the extent caused by occurrences beyond the reasonable control of the party affected, including but not limited to, acts of god, embargoes, governmental restrictions, strikes or other concerted acts of workers, fire, flood, explosion, riots, wars, civil disorder, rebellion or sabotage. The party suffering such occurrence shall immediately notify the other party and any time for performance hereunder shall be extended by the actual time of delay caused by the occurrence.

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## **26. ENTIRE AGREEMENT**

This Agreement and Schedules A and B attached hereto contain the entire agreement and understanding between the parties with respect to the subject matter hereof, and merge all prior discussions, representations and negotiations with respect to the subject matter of this Agreement.

**In Witness Whereof**, the parties hereto have executed this Agreement by their duly authorized officers or representatives.

SALK INSTITUTE FOR BIOLOGICAL STUDIES COMPANY

By: /s/ Anne-Marie Mueller

Anne-Marie Mueller

Senior Director, Office of Technology Management

COMPANY

**Schedule A**  
**PATENT RIGHTS**  
**WPRE**

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**Schedule B**  
**Biological Materials**

WPRE (Woodchuck Hepatitis Virus Posttranscriptional Regulatory Element) as described in *Journal of Virology* Vol. 72, No. 6, p. 5085-5092 (June 1998)

**FIRST AMENDMENT TO LICENSE AGREEMENT ID 2008-0006**

This First Amendment to a License Agreement between the parties dated February 8, 2008 (this “First Amendment”) is effective this XX day of May, 2013 (the “Amendment Date”), by and between the THE SALK INSTITUTE FOR BIOLOGICAL STUDIES, a nonprofit public benefit corporation organized and existing under the laws of the State of California, USA, having an office at 10010 North Torrey Pines Road, La Jolla, CA 92037 (“Salk”) and uniQure biopharma BV (formerly AMSTERDAM MOLECULAR THERAPEUTICS BV), having a principal place of business at P.O. Box 22506 1100 DA Amsterdam The Netherlands (“Licensee”).

**BACKGROUND**

WHEREAS, Salk is the owner of certain patents and biological materials related to RNA Export Element and Methods of Use developed in the laboratory of [\*\*] of the Salk faculty;

WHEREAS, Salk and Licensee executed a License Agreement dated February 8, 2008 (the “License Agreement”) in which Salk granted to Licensee a non-exclusive license to such patents and biological materials as specifically set forth in the License Agreement; and

WHEREAS, effective April 26, 2012, Licensee changed its name from AMSTERDAM MOLECULAR THERAPEUTICS BV to uniQure’s biopharma BV with no change in corporate structure; and

WHEREAS, the parties now desire to make certain changes to the License Agreement, effective as of the Amendment Date.

NOW, THEREFORE, in consideration of the foregoing and for good and valuable consideration, the receipt and sufficiency which are hereby acknowledged, the parties hereby agree as follows:

1. Amendments to Section 1.

(a) Section 1.6. The second line of Section 1.6 of the Agreement is hereby amended and restated as follows:

“sales by Licensee, its Affiliates or Sublicensees to any third party, in the sale of a Licensed Product less: ”

A new sentence is hereby added to the end of Section 1.6 as follows:

“In the event of a sale of a Licensed Product from Licensee to a Sublicensee followed by a subsequent sale of such Licensed Product by the Sublicensee, royalties on Net Sales shall be payable to Salk only once, on the sale that would result in the highest royalty to Salk. ”

(b) Section 1.9. A new Section 1.9 is hereby added to the Agreement as follows:

“ **“Non-Royalty Sublicense Income”** shall mean execution fees, maintenance fees, milestone fees and all other non-royalty payments received by Licensee from its Sublicensees pursuant to any sublicense granted pursuant to Section 2.3 hereunder. ”

(c) Section 1.10. A new Section 1.10 is hereby added to the Agreement as follows:

“ **“Sublicense”** shall mean any agreement or multiple agreements with a non-Affiliate third party in which Licensee grants the third party any of the rights granted to Licensee in Section 3.1. ”

(d) Section 1.11. A new Section 1.11 is hereby added to the Agreement as follows:

“ **“Sublicensee”** shall mean any non-Affiliate third party to whom Licensee has granted a Sublicense. ”

2. Amendments to Section 3.

(a) Section 3.2. Section 3.2 of the Agreement is hereby deleted and replaced as follows:

“ 3.2 Subject to the terms and conditions hereof, Licensee shall have the right to grant Sublicenses under the license granted in Section 3.1 upon prior written approval of each proposed Sublicensee by Salk, not to be unreasonably withheld, provided that (i) no Sublicensee shall have the right to sublicense, (ii) such Sublicenses include a royalty rate upon Sublicensee’s Net Sales in an amount at least equal to the rate set forth in Section 4.3, (iii) Sublicenses entered into by Licensee shall conform in all material respects to the applicable terms and conditions of this Agreement and Sublicensees shall be subject to all applicable restrictions, limitations and obligations imposed on Licensee hereunder, and (iv) the sublicensed rights of any Sublicensee shall terminate upon termination of this Agreement. In the event this License Agreement is terminated or upon the expiration of the Term, such Sublicenses shall at Salk’s sole discretion, which shall not be unreasonably withheld, be automatically become a direct license with Salk. Licensee agrees to forward to Salk a copy of any and all Sublicenses promptly upon execution thereof, but in no event later than [\*\*] days after each such Sublicense has been executed by both parties thereto.”

3. Amendments to Section 4.

(a) Section 4.6. A new Section 4.6 is hereby added to the Agreement as follows:

“4.6 Licensee shall pay Salk a Sublicense fee, nonrefundable and noncreditable against royalties, of Five Thousand Dollars (\$5,000.00), due within [\*\*] business days from the Effective Date of this First Amendment. ”

(b) Section 4.7. A new Section 4.7 is hereby added to the Agreement as follows:

“ 4.7 Licensee shall pay Salk on a quarterly basis a share of Non-Royalty Sublicense Income of [\*\*] percent ([\*\*]%). ”

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4. Amendments to Section 5.

(a) Section 5.1. Section 5.1 of the Agreement is hereby deleted and replaced as follows:

“5.1 Licensee agrees to keep, and agrees to require each of its Sublicensees to keep, proper records of scientific research and keep books of account in accordance with international financial reporting standards. Such records and books shall include all information necessary for the accurate determination of royalty and other payments owed to Salk. Following launch of any Licensed Product, Licensee, on behalf of itself, its Affiliates and its Sublicensees, agrees to deliver to Salk, within [\*\*] days after each calendar quarter, a report showing the information on which payments herein provided are calculated, including a breakdown of income from sales of each Licensed Product, and to accompany each such report with the payment shown to be due thereby. All amounts accrued for the benefit of Salk shall be deemed held in trust for the benefit of Salk until each payment of such amounts is made pursuant to this Agreement. ”

(b) Section 5.4 . Section 5.4 of the Agreement is hereby deleted and replaced as follows:

“ 5.4 On reasonable written notice, Salk, at its own expense, shall have the right to have an independent party reasonably acceptable to the Licensee or its Sublicensee, as applicable, inspect and audit the books and records of Licensee, its Affiliates and any Sublicensee at their offices during usual business hours for the sole purpose of, and only to the extent necessary for, determining the correctness of payments due under this Agreement. Such examination with respect to any fiscal year shall not take place later than [\*\*] years following the expiration or termination of this Agreement. The expense of any such audit shall be borne by Salk; provided, however, that if the audit discloses an error in excess of [\*\*] percent ([\*\*]%) in favor of Licensee, then Licensee shall pay, in addition to the amount of any underpayment, the cost to Salk of the audit. ”

5. Amendments to Section 6.

(a) Section 6.3. Section 6.3 of the Agreement is hereby deleted and replaced as follows:

“6.3 Licensee shall notify Salk of any improvements (including, but not limited to, modified derivatives and variants) to the Biological Materials made by Licensee, its Affiliates, or Sublicensees, irrespective of whether any such improvements were made in collaboration with Salk or any of its employees. At Salk’s request, Licensee shall provide Salk with reasonable quantities of such improvements, and Salk scientists shall have the right to use them for their internal research. Title to all such improvements shall remain with Licensee, its Affiliates or Sublicensees, as applicable. ”

6. Amendments to Section 7.

Section 7 of the Agreement is hereby deleted and replaced as follows:

“ Licensee (for itself and its Affiliates) acknowledges and agrees, and will require any Sublicensees to acknowledge and agree, that Salk is and shall remain (as to Licensee, its Affiliates and any Sublicensees) the owner of the Licensed Patents and Biological Materials,

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subject to the rights of the Federal Government as set forth in 35 U.S.C. §200, et seq., and that neither Licensee nor its Affiliates or Sublicensees has any rights in or to the Licensed Patents and Biological Materials other than the rights specifically granted herein. All rights not expressly granted in this Agreement are reserved to Salk. ”

7. Amendments to Section 9.

(a) Section 9.3(b)(i). Section 9.3(b)(i) of the Agreement is hereby deleted and replaced as follows:

“ (i) Neither Licensee, its Affiliates or its Sublicensees shall thereby be discharged from any liability or obligation to Salk which became due or payable prior to the effective date of such expiration or termination; and ”

(b) Section 9.3(b)(iii). Section 9.3(b)(iii) of the Agreement is hereby deleted and replaced as follows:

“(iii) Licensee shall, and shall ensure that its Affiliates and Sublicensees, promptly return all materials, samples, documents, information, and other materials which embody or disclose Licensed Technology; provided, however, that Licensee, its Affiliates and its Sublicensees shall not be obligated to provide Salk with proprietary information which Licensee, its Affiliate and its Sublicensee, as applicable, can show that it independently developed. Licensee, its Affiliates and its Sublicensees shall be permitted to retain copies of technical information in documentary form obtained from Salk where required to do so by reason of any statute, ordinance or regulation of any federal, state, or local government entity.”

(c) Section 9.3(c). Section 9.3(c)(i) of the Agreement is hereby deleted and replaced as follows:

“(i) Licensee agrees, and shall require its Affiliates and Sublicensees to agree, not to use Licensed Technology or said technical information after termination of this Agreement. If at termination Licensee, its Affiliates, or Sublicensees then possess Licensed Product, have started the manufacture thereof or have accepted orders therefor, Licensee, its Affiliates, or Sublicensees shall have the right to sell their inventories thereof, complete the manufacture thereof and market such fully manufactured Licensed Product, in order to fulfill such accepted orders, subject to the obligation of Licensee, its Affiliates, and Sublicensees to pay Salk the royalty payments therefor as provided in Paragraph 4 of this Agreement; ”

(d) Section 9.3(c)(ii). Section 9.3(c)(ii) of the Agreement is hereby deleted and replaced as follows:

“ (ii) Subject to Paragraph 9.3(c)(i), Licensee shall, and shall require its Affiliates and Sublicensees to, discontinue the manufacture, use, marketing and sale of Licensed Products;”

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(e) Section 9.3(c)(iii). Section 9.3(c)(iii) of the Agreement is hereby deleted and replaced as follows:

“(iii) Licensee, on behalf of itself, its Affiliates and its Sublicensees, shall provide a final report of the type described in Paragraph 5.1, including any allowable post-termination sales; and ”

8. Amendments to Section 13.

(a) Section 13.3. Section 13.3 of the Agreement is hereby deleted and replaced as follows:

“13.3 IN NO EVENT WILL SALK BE LIABLE TO LICENSEE, ITS AFFILIATES, ITS SUBLICENSEES OR ANY THIRD PARTY FOR ANY CONSEQUENTIAL, INDIRECT, PUNITIVE, EXEMPLARY, SPECIAL, OR INCIDENTAL DAMAGES, INCLUDING ANY LOST PROFITS OR LOSS OF DATA, ARISING FROM OR RELATING TO THIS AGREEMENT OR THE LICENSED TECHNOLOGY. SALK’S TOTAL CUMULATIVE LIABILITY IN CONNECTION WITH THIS AGREEMENT AND THE LICENSED TECHNOLOGY, WHETHER IN CONTRACT OR TORT OR OTHERWISE, WILL NOT EXCEED THE AMOUNTS PAID TO SALK UNDER THIS AGREEMENT WITHIN TWELVE (12) MONTHS PRECEDING THE CLAIM. ”

9. Amendments to Section 14.

(a) Section 14.1. Section 14.1 of the Agreement is hereby deleted and replaced as follows:

“ 14.1 Licensee agrees to indemnify, hold harmless and defend Salk, its trustees, officers, employees and agents, the sponsors of the research that led to the Licensed Technology, and the inventors of the patents and patent applications included in the Licensed Patents (hereinafter the “Indemnitees”) against any and all liability and/or damages with respect to any claims, suits, demands, judgments or causes of action arising out of (a) the research, development, manufacture, storage, sale or other distribution, or any other use of Licensed Products or Licensed Technology, or exercise of rights granted hereunder, by Licensee, its Affiliates, its Sublicensees, or its distributors, agents or representatives; (b) the use by end-users and other third parties of Licensed Products or Licensed Technology; and/or (c) any representation, warranty or statement by Licensee or its Affiliates, Sublicensees, distributors, agents or representatives, concerning the Indemnitees, the Licensed Technology or Licensed Products. In the event any such claims, demands or actions are made. Licensee shall defend the Indemnitees at Licensee’s sole expense by counsel selected by Licensee, subject to approval by Salk, which is not to be unreasonably withheld. No settlement, consent judgment or other voluntary final disposition may be entered into without the prior written consent of Salk, which consent shall not be unreasonably withheld.”

(b) Section 14.2. Section 14.2 of the Agreement is hereby deleted and replaced as follows:

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“ 14.2 Licensee shall maintain, and shall require its Affiliates and Sublicensees to maintain, continuously and without interruption during the Term (as defined in Section 9.1), comprehensive general liability insurance, including products liability insurance, with reputable and financially secure insurance carriers to cover the activities of Licensee, its Affiliates and its Sublicensees. Such insurance (a) shall include endorsements naming Salk as an additional insured and waiving any right of subrogation against Salk, (b) shall require prior notice to Salk before cancellation, and (c) shall be written to cover claims incurred, discovered, manifested, or made during or after the expiration of this Agreement. Such insurance shall have a minimum limit of [\*\*] dollars (\$[\*\*]) per specific occurrence and a minimum limit of [\*\*] dollars (\$[\*\*]) for aggregate liability insurance; provided, however, that not less than

[\*\*] days before the earlier date upon which Licensee or its Affiliates or Sublicensees (i) initiates testing of Licensed Products in a clinical trial involving human subjects for purposes of diagnosis or treatment, or (ii) makes a commercial sale of any Licensed Product, such coverage shall be increased to a minimum limit of [\*\*] dollars (\$[\*\*]) per specific occurrence and a minimum limit of [\*\*] dollars (\$[\*\*]) for aggregate liability insurance. Licensee shall maintain insurance as required by this Section 14.2 beyond the expiration or termination of this Agreement during any period that any product, process, or service, relating to, or developed pursuant to, this Agreement is being commercially distributed or sold by Licensee or a Sublicensee, Affiliate or agent of Licensee and thereafter for a period of [\*\*] years. The minimum amounts of insurance coverage required shall not be construed to create a limit of Licensee’s liability with respect to its indemnification under this Agreement. Failure of Licensee to comply with the requirements of this Section 14.2 shall be considered a material breach of the Agreement. ”

(c) Section 14.3. A new Section 14.3 is hereby added to the Agreement as follows:

“14.3 Licensee shall furnish a certificate of insurance evidencing primary coverage and additional insured requirements and provide Salk with copies of subsequent annual certificates of insurance. Licensee shall provide Salk with written notice at least [\*\*] days prior to the cancellation, non-renewal or material change in such insurance; if Licensee does not obtain replacement insurance providing comparable coverage within such [\*\*] day period, Salk shall have the right to terminate this Agreement effective at the end of such [\*\*] day period without notice or any additional waiting periods. ”

10. Amendments to Section 16. Section 16 of the Agreement is hereby deleted and replaced as follows:

“16. To comply with US Government regulations for the licensing of federally funded inventions, Licensee, its Affiliates and its Sublicensees will commit that Licensed Products sold in the US will be manufactured substantially in the US. Notwithstanding the foregoing, if during the term of the License Agreement Licensee, its Affiliates and/or its Sublicensees can provide reasonably compelling evidence to Salk that such manufacture in the US would impose an extraordinary burden on Licensee, its Affiliate or any Sublicensee, Salk shall at that time agree to seek a waiver from the US Government with respect to the requirement that Licensed Products or Licensed Services for sale in the US be manufactured substantially in the US. Licensee understands that Salk cannot

guarantee that such waiver can be obtained. Licensee shall bear all costs associated with seeking such waiver. ”

11. Schedule A. Schedule A of the Agreement is hereby deleted and replaced in its entirety by the attached, amended Schedule A.

12. Miscellaneous.

- a. Except as specifically amended above, all terms of the Agreement shall remain in full force and effect To the extent that there are any inconsistencies between the terms of the Agreement and the terms of this First Amendment, the terms of this First Amendment shall prevail.
- b. The parties acknowledge that this First Amendment and the Agreement set forth the entire understanding and intentions of the parties hereto as to the subject matter hereof and supersedes all previous understandings between the parties, written or oral, regarding such subject matter.

IN WITNESS WHEREOF, the parties have executed this First Amendment as of the Amendment Date.

SALK INSTITUTE FOR BIOLOGIC STUDIES

By /s/ Paul Roben  
Name: Paul Roben  
Title: Senior Director,  
Office of Technology Development

LICENSEE

By \_\_\_\_\_  
Name:  
Title:

Schedule A

PATENT RIGHTS

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Confidential Materials omitted and filed separately with the Securities and Exchange Commission. Double asterisks denote omissions.

## LICENSE AGREEMENT

THIS AGREEMENT (the “Agreement”) is made and is effective December 5, 2006, (the “Effective Date”) by and between Targeted Genetics Corporation, a corporation having a principal place of business at 1100 Olive Way, Suite 100, Seattle, Washington 98101 (“TGC”), and Amsterdam Molecular Therapeutics B.V. (“AMT”), a corporation having a principal place of business at Meibergdreef 61, 1100 DA Amsterdam, The Netherlands.

## RECITALS

WHEREAS, TGC has exclusively licensed the Licensed Patent Rights (relating to an AAV1 Vector gene delivery system) from the University of Pennsylvania as part of a license agreement entered into between TGC and the University of Pennsylvania (“UPenn”) with the effective date of June 1, 2002 (“UPenn Agreement”).

WHEREAS, AMT is developing an AAV1 product to treat LPL type 1 and LPL type 5 deficiency that requires a license to the Licensed Patent Rights and therefore seeks a license to the Licensed Patent Rights in the Field, as such terms are defined herein; and

WHEREAS, TGC is willing to grant such a license on the terms set forth herein and the University of Pennsylvania is willing to acknowledge such grant of a sublicense.

NOW THEREFORE, the parties agree as follows:

## 1. DEFINITIONS

1.1 “AAV1 Vector” means the adeno-associated virus serotype 1 vector technology which includes without limitation the AAV serotype 1 rep, cap, and ITR sequences and proteins whether utilized in whole or in part to deliver therapeutic genes into cells, and which is the subject of the Licensed Patent Rights

1.2 “Affiliate” means any company or other legal entity other than AMT in whatever country organized, controlling, controlled by or under common control with AMT. The term “control” means possession, direct or indirect, of the powers to direct, cause or significantly influence the direction of the management and policies of the company or entity in question, whether through the ownership of voting securities, by contract or otherwise.

1.3 “Federal Government Interest” means the rights of the United States Government under Public Laws 96-517, 97-256 and 98-620, codified at 35 U.S.C. 200-212, and any regulations issued thereunder, as such statute or regulations may be amended from time to time hereafter.

1.4 “Field” means treatment of LPL deficiency type 1 and LPL deficiency type 5 by in vivo gene therapy utilizing an AAV1 Vector encoding the LPL gene.

1.5 “Included CIPs” has the meaning given in Paragraph 1.9.

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1.6 “License” has the meaning given in Paragraph 2.1.

1.7 “LPL” means Lipoprotein Lipase.

1.8 “Licensed Patent Rights” means rights to any subject matter described or claimed in U.S. Patent Nos. [\*\*]; and all foreign counterparts including all continuations, divisional, and any continuation-in-part applications, to the extent that such continuation-in-part (or any continuation or divisional thereof) has claims directed to subject matter enabled and described in U.S. Patent Nos. [\*\*] and such claims are necessary or relevant for the Field (collectively, “Included CIPs”); any patents issuing on said applications, continuing applications, divisional applications, including reissues and reexaminations thereof, and any foreign applications or patents corresponding directly thereto.

1.9 “Licensed Product” means a product in the Field that, absent the License, would in the country of sale infringe any Valid Claim within the Licensed Patent Rights in such country.

1.10 “Net Sales” means (a) the actual gross invoice price of Licensed Products sold by AMT or its Affiliates to any Third Party, less, to the extent included therein, the total of (i) ordinary and customary trade discounts; (ii) sales and excise taxes, and other similar taxes, customs duty and compulsory payments to governmental authorities actually paid or deducted and related to the sale (excluding what is commonly known as income taxes); and (iii) credits given to customers for rejects or returns of the product. For purposes hereof, Net Sales shall not include sales of a Licensed Product from AMT to an Affiliate of AMT; it being intended that Net Sales shall only include sales to unrelated third-parties.

1.11 “Person” means an individual, sole proprietorship, partnership, limited partnership, limited liability partnership, corporation, limited liability company, business trust, joint stock company, trust, unincorporated association, joint venture, or other similar entity or organization, including without limitation, a government or political subdivision, department or agency of a government.

1.12 “Royalties” has the meaning given in Paragraph 4.1.

1.13 “Third Party” means any Person other than AMT, TGC or any of their respective Affiliates.

1.14 “Valid Claim” means

i) a claim of an issued and unexpired patent included within Licensed Patent Rights, which has not been held permanently revoked, unenforceable or invalid by a decision of a court or other governmental agency of competent jurisdiction, unappealable or unappealed within the time allowed for

appeal, and which has not been admitted to be invalid or unenforceable through reissue or disclaimer or otherwise; or

ii) a claim of a pending patent application included within Licensed Patent Rights which claim was filed and is being prosecuted in good faith and has not been abandoned

or finally disallowed without the possibility of appeal or refiling of the application, provided that no more than [\*\*] years have passed since the earliest priority date for such application.

2. GRANT

2.1 TGC grants to AMT a non-exclusive, non-sublicensable license under the Licensed Patent Rights in each country of the world where there are Valid Claims of Licensed Patent Rights, to make, have made, develop, use, sell, offer to sell and import Licensed Products (the “License”).

2.2 AMT acknowledges that in accordance with the Federal Government Interest, the United States government retains certain rights in intellectual property funded in whole or part under any contract, grant or similar agreement with a Federal agency, including but not limited to the requirement that Licensed Products subject to sale in the United States must be substantially manufactured in the United States. The license grant of this Article 2 is expressly subject to all of such rights.

2.3 UPenn retains the reserved right to use, and to permit other for-profit or nonprofit organizations to use, the Licensed Patent Rights strictly for educational and for research purposes. Any such rights of UPenn or a third party to practice the Licensed Patent Rights for educational or research purposes shall be royalty-free.

3. CONSIDERATION AND MAINTENANCE FEES

3.1 Upon signing this Agreement, AMT shall pay to TGC a \$1,750,000 fee in consideration of the License.

3.2 Upon the first anniversary of the Effective Date and upon each anniversary during the Term thereafter, AMT shall pay to TGC a \$100,000 maintenance fee for maintenance of the License.

4. ROYALTIES AND PAYMENTS

4.1 AMT shall pay to TGC earned royalties based on Net Sales (“Royalties”) in the amount of: (i) [\*\*] percent ([\*\*]%) of the Net Sales of Licensed Products if cumulative Net Sales are less than \$[\*\*], or (ii) [\*\*] percent ([\*\*]%) of the Net Sales of Licensed Products if cumulative Net Sales are equal to or greater than \$[\*\*] but less than \$[\*\*], or (iii) [\*\*] percent ([\*\*]%) of Net Sales of Licensed Products if cumulative Net Sales are \$[\*\*] or more. In the case other third party royalties are paid by AMT to Third Party licensors of intellectual property that is required for the composition of AAV1 , then [\*\*] percent ([\*\*]%) of such royalty actually paid to Third Parties shall be deducted from the royalty in 4.1(i), (ii) or (iii), with a minimum due to TGC of [\*\*] percent ([\*\*]%) if cumulative Net Sales of Licensed Products are less than \$[\*\*] or, [\*\*] percent ([\*\*]%) if the cumulative Net Sales of Licensed Products are greater than or equal to \$[\*\*] but less than \$[\*\*] or, [\*\*]percent ([\*\*]%) if the cumulative Net Sales of Licensed Products are greater than or equal to \$[\*\*]. The Net Sales thresholds in such section (i)-(iii) will be applied separately to each distinct Licensed Product, but where the same Licensed Product is packaged or labeled differently in different nations, or otherwise to accommodate the same to

different markets or indications, the Net Sales thereof in all such nations, markets and indications shall be aggregated.

4.2 In addition to the Royalties and other payments set forth herein, AMT shall pay TGC the following “Milestone Payments.” For the purposes of this Agreement, the [\*\*]. For the purpose of clarity, the total payments for milestones achieved assuming both the LPL type 1 and 5 deficiency proceed through regulatory approval for marketing would be four million nine hundred and fifty thousand U.S. Dollars (\$4,950,000):

Section	Milestone	Milestone Payments
4.2.1	[**]	[**]
4.2.2	[**]	[**]
4.2.3	[**]	[**]
4.2.4	[**]	[**]
4.2.5	Upon first regulatory approval (BLA or other) for each indication	[**]

4.3 Royalties accruing to TGC shall be paid to TGC on a quarterly basis. Each such payment will be for royalties which accrued within the most recently completed calendar quarter and payment shall be made by AMT within [\*\*] days of the end of such calendar quarter.

4.4 Milestone Payments shall be paid to TGC within [\*\*] days of achievement.

4.5 Royalty or Milestone Amounts that are not paid when due shall accrue interest from the due date until paid, at a rate equal to [\*\*] percent ([\*\*]%) per month (or the maximum allowed by law, if less).

4.6 AMT must maintain complete and accurate books and records which enable the Royalties, fees, and payments payable under this Agreement to be verified. The records for each calendar quarter must be maintained for [\*\*] years after the submission of each report under Article 4. Upon reasonable prior written notice to AMT from TGC, AMT shall permit a certified public accountant or a person possessing similar professional status and associated with an



independent accounting firm reasonably acceptable to AMT to inspect all books and records relating to the sales of Licensed Products by AMT as necessary to verify the same. Access to these books and records pertaining to Net Sales must be made available no more than [\*\*] for each Licensed Product, during normal business hours, and [\*\*] for each Licensed Product during each of the [\*\*] years after expiration or termination of this Agreement. The accounting firm shall enter into appropriate obligations with AMT to treat all information it receives during its inspection in confidence. The accounting firm shall disclose to the parties only whether such books and records are correct and details concerning any discrepancies, but no other information shall be disclosed to TGC. The charges of the accounting firm shall be paid by TGC, except if a review or audit of such books and records of AMT determines that AMT has underpaid royalties

on Licensed Products by [\*\*] percent ([\*\*]%) or more then AMT must pay the charges of the accounting firm in connection with such review or audit. Notwithstanding the foregoing, AMT agrees to conduct, at its expense, an independent audit of sales and Royalties with respect to a Licensed Product at least every [\*\*] years once annual sales of such Licensed Product are greater than [\*\*] dollars (\$[\*\*]) per annum. The audit shall address, at a minimum, the amount of gross sales by or on behalf of AMT during the audit period, the amount of funds owed to TGC under this Agreement, and whether the amount owed has been paid to TGC and is reflected in the records of the AMT. A report by the auditors shall be submitted promptly to TGC upon completion.

4.7 In the event that any patent or any claim thereof included within the Licensed Patent Rights shall be held invalid or unenforceable in a final decision by a court of competent jurisdiction from which no appeal has or can be taken, any and all obligation to pay Royalties based solely on such patent or claim shall cease as of the date of such final decision.

4.8 Any component of Net Sales denominated in currencies other than U.S. Dollars shall be converted into U.S. Dollars in accordance with the provisions of this paragraph, and reported in U.S. Dollars. All payments required under this Agreement from time to time shall be made in U.S. Dollars. Any currency conversions shall be made using the average quarterly exchange rates published regularly by Citibank, New York, or its successor. The average will be calculated by summing the exchange rates for the final business day of each of the three (3) months in the applicable calendar quarter and dividing by three (3). All currency conversions will be calculated to an accuracy of three (3) digits after the decimal point.

## **5. PATENT FILING, PROSECUTION AND MAINTENANCE**

5.1 As between the parties, TGC shall have the responsibility for the preparation, filing, prosecution and maintenance of the Licensed Patent Rights in the United States and foreign countries for which patent protection has been sought. TGC shall maintain the Licensed Patent Rights in all territories for the maximum time period permitted by applicable law, including by timely paying all applicable maintenance fees and diligently prosecuting any patent applications and diligently defending any Third Party challenges to validity or enforceability.

## **6. PATENT INFRINGEMENT**

6.1 In the event that AMT learns of the infringement of any Licensed Patent Rights by the manufacture, use or sale of a product in the Field, AMT shall so inform TGC in writing and shall provide TGC with reasonable evidence of such infringement.

6.2 As between the parties, TGC shall have the first right but not the obligation to prosecute such infringement of the Licensed Patent Rights. In the event such infringement relates specifically to the manufacture, use or sale of a product in the Field, AMT shall have the right but not the obligation to participate in such infringement litigation and be represented by counsel of its choice at its own expense. In the event TGC notifies AMT that it will not bring such action as it relates specifically to the manufacture, use or sale of a product in the Field, AMT at its own expense shall have the right to bring such action and shall cause TGC to be

joined in such action in jurisdictions where it is necessary for TGC to be named in order for AMT to have standing in such infringement litigations; provided, however, that AMT shall allow TGC to participate and be represented by counsel of its own choice at AMT's sole expense. AMT shall not nor shall AMT cause TGC to settle or compromise any such suit in a manner that imposes any obligations or restrictions on TGC or grants any License Patent Rights without TGC's written permission.

6.3 Each party shall, at the request and expense of the party initiating such suit, cooperate in all respects, including being joined as a named party, and, to the extent possible, have its employees testify when requested and make available relevant records, papers, information, samples, specimens, and the like. No such suit shall be settled by a party in any manner which diminishes the rights of the other party hereto under this Agreement without the written agreement of both parties.

6.4 Any legal action against a third party under this Section 6 shall be at the expense of the party on account of whom suit is brought and recoveries recovered thereby shall first be allocated to reimburse the expenses of the parties on a pro rata basis, after which, when the infringement relates to the manufacture, use or sale of a product in the Field, any remaining recoveries shall belong to AMT and AMT shall pay TGC royalty amounts set forth in Section 4 on such remaining recoveries as if such recoveries were Net Sales.

## **7. REPORTING**

7.1 AMT will make quarterly royalty reports to TGC on or before each [\*\*] of each year (i.e., within [\*\*] days from the end of each calendar quarter) and certified by the chief financial officer of AMT. Each such royalty report will cover AMT's most recently completed calendar quarter and will show: (a) the gross sales and Net Sales of Licensed Products sold by AMT during the most recently completed calendar quarter, including (i) all amounts invoiced, billed, or received; and (ii) the number of units and the country of sale; and (b) the Royalties payable hereunder with respect to such. If no sales of Licensed Products have been made during any reporting period, a statement to this effect shall be provided by AMT to TGC.

### **7.2 Progress Reports.**

7.2.1 AMT will provide TGC with a written plan relating to AMT's development in the Field (the "Development Plan") within [\*\*] days after the signing of this Agreement by both parties. The Development Plan will outline the disease indications, patient populations to be addressed, publicly available information on competition and estimates of development timelines for AMT's products in the Field. The Development Plan will separately address activities applicable to each Licensed Product.

7.2.2 At or within [\*\*] days following each anniversary of the Effective Date, AMT will provide TGC with a written progress report that describes any progress made against the Development Plan (as supplemented by progress reports, where applicable) submitted a year earlier and plans for development in the coming year. AMT shall also notify TGC within [\*\*] days of the first commercial sale of any Licensed Product.

7.2.3 AMT will provide TGC with audited financial statements, produced by a certified public accounting firm, [\*\*] days following the end of each of AMT's fiscal years, as well as a copy of any management letter recommendations submitted by the auditors.

7.2.4 Where applicable AMT will provide TGC with copies of reports such as Form 10-K and Form 10-Q filings made to the United States Securities and Exchange Commission or other similar regulatory agency outside of the United States.

7.2.5 TGC shall keep the Development Plan, all such progress reports, financial statements and any other information AMT provided to TGC under this Section 7 confidential, other than the provision of such reports to the University of Pennsylvania as required under the UPenn Agreement, for five (5) years or until the time, if any, of the first commercial sale of any Licensed Product(s).

## **8. TERM OF THE AGREEMENT**

8.1 Unless otherwise terminated in accordance with the terms of this Agreement, this Agreement shall be in force from the Effective Date until the last to expire Valid Claim of Licensed Patent Rights (the "Term").

8.2 Any termination of this Agreement shall not affect the rights and obligations set forth in the following Articles:

Article 6.4 Allocation of Patent Infringement Recovery  
Article 11.2 Disposition of Licensed Products on Hand upon Termination  
Article 13 Use of Names and Trademarks  
Article 15 Limitation of Liability  
Article 16 Indemnification  
Article 19 Failure to Perform  
Article 20 Governing Law

## **9. TERMINATION BY TGC**

9.1 If AMT violates or fails to pay Royalties, Milestone Payments or fees under Articles 3 or 4, or (ii) breaches or fails to perform, or has any other default, under any contractual obligation of AMT to TGC and fails to repair any default in Sections 9.1(i) or 9.1(ii) within [\*\*] days after receipt of such notice of breach, TGC shall have the right to terminate this Agreement by a second written notice ("Notice of Termination") to AMT. If a Notice of Termination is sent to AMT, this Agreement shall terminate fifteen (15) days after receipt of such notice unless other terms are mutually agreed upon.

9.2 If AMT shall cease its operations other than in connection with a reorganization of its business in connection with a redomociliation or other corporate event unrelated to the solvency of AMT, become bankrupt or insolvent, apply for or consent to the appointment of a trustee, receiver or liquidator of its assets or seek relief under any law for the aid of debtors then TGC shall have the right to terminate the License.

## **10. TERMINATION BY AMT**

10.1 AMT shall have the right to terminate this Agreement at any time, effective sixty (60) days after receipt by TGC of a written notice of termination delivered pursuant to this section.

10.2 AMT may terminate this Agreement with immediate effect by giving written notice to TGC, if TGC ceases its operations, becomes insolvent, applies for or consents to the appointment of a trustee, receiver or liquidator of its assets, or seeks relief under any law for the aid of debtors. If TGC violates or fails to perform any material term or covenant of this Agreement, then AMT may give written notice of such default ("Notice of Default") to TGC. If TGC fails to repair such default within [\*\*] days after receipt of such Notice of Default, AMT shall have the right to terminate this Agreement by a Notice of Termination to TGC, effective immediately.

## **11. EFFECT OF TERMINATION, DISPOSITION OF LICENSED PRODUCTS UPON TERMINATION**

11.1 Effect of Termination. Upon the termination of this Agreement, except pursuant to Paragraph 10.2, the License granted by TGC to AMT hereunder shall revert to TGC.

11.2 Upon termination of this Agreement for any reason, except pursuant to Paragraph 10.2, AMT shall have the right to dispose of all previously made or partially made Licensed Products, within a period of [\*\*] months; provided, however, that the sale of such Licensed Products shall be subject to the terms of this Agreement including, but not limited to, the payment of Royalties at the rate and at the time provided herein and the rendering of reports.

11.3 Upon termination of this Agreement for any reason, except pursuant to Paragraph 10.2, AMT shall pay, within [\*\*] days to TGC, pursuant to Section 3 and 4, any and all amounts due within [\*\*] days of receipt by TGC of a notice of termination from AMT.

11.4 Upon the termination of this Agreement pursuant to Paragraph 10.2 or the termination of the UPenn Agreement, TGC's rights hereunder shall be assigned directly to UPenn and shall remain in full force and effect under the terms hereof; and, provided that AMT is not in material breach of this Agreement, the rights and obligations of AMT herein (including but not limited to the License) shall continue in full force and effect without expansion, restriction, inhibition or diminution; provided that UPenn will require execution of an amended and restated license with AMT that will (i) restate Article 6, Patent Infringement, to reflect the corresponding provisions of UPenn's then standard non-exclusive patent license agreement, (ii) limit any representations or warranties by UPenn under

Article 14 of this Agreement to the representations made in Section 9.5 of the UPenn Agreement, (iii) omit Article 23 of this Agreement, and (iv) include Sections 12.1, 12.9 and 12.10 of the UPenn Agreement, substituting “AMT” in place of “Targeted” in each instance, see attachment A. For clarity, the financial terms of this Agreement will remain unaltered.

11.5 Nothing herein shall constitute a waiver of either party’s right to seek damages in the event of a material breach of this Agreement by the other party.

## **12. PATENT MARKING**

12.1 AMT agrees to mark all Licensed Products made, used or sold under the terms of this Agreement, or their containers, in accordance with the applicable patent marking laws.

## **13. USE OF NAMES AND TRADEMARKS**

13.1 Unless required by law, nothing contained in this Agreement shall be construed as conferring any right to use in advertising, publicity, or other promotional activities any name, trade name, trademark, or other designation of either party hereto or the UPenn (including any contraction or abbreviation of the foregoing).

13.2 Unless required by law, neither party shall disclose, at any time, the financial terms or events upon which financial obligations are triggered in the License, save that TGC and AMT shall have the right to do so (i) to the extent such disclosure is required in publicly filed financial statements or other public statements under rules governing a stock exchange (e.g., the rules of the United States Securities and Exchange Commission, NASDAQ, NYSE, Amsterdam, UKLA or any other stock exchange on which securities issued by either Party may be listed); and (ii) to its actual or potential investment bankers; (iii) to existing and potential investors in connection with an offering or placement of securities for purposes of obtaining financing for its business and to actual and prospective lenders for the purpose of obtaining financing for its business; and (iv) to a bona fide potential acquiror or merger partner for the purposes of evaluating entering into a merger or acquisition, provided, however, any such persons must be obligated to hold in confidence and not make use of such confidential information for any purpose. A press release acknowledging the grant of the License by TGC to AMT shall be publicly disclosed by either or both parties, only upon mutual agreement of the text of such release by both parties.

## **14. REPRESENTATIONS, WARRANTIES AND COVENANTS**

14.1 Each party hereby represents, warrants, and covenants to the other party as of the Effective Date as follows:

i) such party (i) has the power and authority and the legal right to enter into this Agreement and perform its obligations hereunder, and (ii) has taken all necessary action on its part required to authorize the execution and delivery of this Agreement and the performance of its obligations hereunder. This Agreement has been duly executed and delivered on behalf of such party and constitutes a legal, valid, binding obligation of such party and is enforceable against it in accordance with its terms;

ii) such party is not aware of any pending or threatened litigation (and has not received any communication) that alleges that such party’s activities related to this Agreement have violated, or that by conducting the activities as contemplated herein such party would violate, any of the intellectual property rights of any other Person;

iii) all necessary consents, approvals and authorizations of all governmental authorities and other Persons or entities required to be obtained by such party in connection with this Agreement have been obtained; and

14.2 TGC hereby represents, warrants, and covenants to AMT as of the Effective Date that TGC is the exclusive licensee of the Licensed Patent Rights. During the term of this Agreement, TGC shall use its best efforts not to encumber or diminish the rights granted to AMT hereunder, including, without limitation, by not committing any acts or permitting the occurrence of any omissions that would cause the breach or termination of the UPenn Agreement. TGC shall promptly provide AMT with notice of any alleged breach or termination of the UPenn Agreement. As of the date hereof, TGC is not in breach of the UPenn Agreement, and it is in full force and effect.

## **15. DISCLAIMER OF WARRANTY; INDEMNIFICATION**

15.1 THE LICENSED PATENT RIGHTS AND LICENSED PRODUCTS ARE PROVIDED ON AN “AS IS” BASIS AND TGC MAKES NO REPRESENTATIONS OR WARRANTIES, EXPRESS OR IMPLIED, WITH RESPECT THERETO. BY WAY OF EXAMPLE BUT NOT OF LIMITATION, TGC MAKES NO REPRESENTATION OR WARRANTY; (i) OF COMMERCIAL UTILITY; (ii) OF MERCHANTABILITY OR FITNESS FOR A PARTICULAR PURPOSE; OR (iii) THAT THE USE OF THE LICENSED PATENT RIGHTS AND LICENSED PRODUCTS UNDER THIS AGREEMENT WILL NOT INFRINGE ANY PATENT, COPYRIGHT OR TRADEMARK OR OTHER PROPRIETARY OR PROPERTY RIGHTS OF OTHERS. TGC SHALL NOT BE LIABLE TO AMT OR ANY THIRD PARTY WITH RESPECT TO: ANY CLAIM ARISING FROM THE USE OF THE LICENSED PATENT RIGHTS AND LICENSED PRODUCTS LICENSED UNDER THIS AGREEMENT OR FROM THE MANUFACTURE, USE OR SALE OF LICENSED PRODUCTS; OR ANY CLAIM FOR LOSS OF PROFITS, LOSS OR INTERRUPTION OF BUSINESS, OR FOR INDIRECT, SPECIAL OR CONSEQUENTIAL DAMAGES OF ANY KIND.

15.2 AMT will defend, indemnify and hold harmless TGC and its trustees, officers, agents and employees and UPenn and its trustees, officers, agents and employees both collectively and severally (individually, an “Indemnified Party”, and collectively, the “Indemnified Parties”), from and against any and all liability, loss, damage, action, claim or expense suffered or incurred by the Indemnified Parties (including attorney’s fees) (individually, a “Liability”, and collectively, the “Liabilities”) that results from or arises out of (a) the development, use, manufacture, promotion, sale or other disposition of any Licensed Product by AMT or its collaborators or distributors; and (b) any breach by AMT of any covenant or agreement contained in this Agreement; and (c) the enforcement by an Indemnified Party of its rights under this Section. Without limiting the foregoing, AMT will defend, indemnify and hold harmless the Indemnified Parties from and against any Liabilities resulting from:

15.2.1 any product liability or other claim of any kind related to the use by a third party of a Licensed Product that was manufactured, sold or otherwise disposed of by AMT, or its collaborators or distributors, pursuant to and within the scope of such relationships;

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15.2.2 a claim by a third party that the use by AMT or its collaborators or distributors, pursuant to and within the scope of such relationships, of Licensed Patent Rights or the design, composition, manufacture, use, sale or other disposition of any Licensed Product by AMT infringes or violates any patent, copyright, trademark or other intellectual property rights of such third party; and

15.2.3 clinical trials or studies conducted by or on behalf of AMT, or its collaborators or distributors, pursuant to and within the scope of such relationships, relating to the Licensed Products and Licensed Patent Rights, including, without limitation, any claim by or on behalf of a human subject of any such clinical trial or study, any claim arising from the procedures specified in any protocol used in any such clinical trial or study, any claim of deviation, authorized or unauthorized, from the protocols of any such clinical trial or study, and any claim resulting from or arising out of the manufacture or quality control by a third party of any substance administered in any clinical trial or study.

Notwithstanding the foregoing, however, the Liabilities shall not include, and in no instance shall AMT be required to indemnify any Indemnified Party with respect to, any liability, claims, lawsuits, losses, damages, costs or expenses to the extent the same are determined to be the result of any Indemnified Party's gross negligence or willful misconduct.

15.3 The Indemnified Party shall promptly notify AMT of any claim or action giving rise to Liabilities subject to the provisions of the foregoing Section. AMT shall have the right to defend or to cause to be defended any such claim or action, at its cost and expense. AMT shall not settle or compromise any such claim or action in a manner that imposes any restrictions or obligations on TGC or grants any rights to Licensed Patent Rights or Licensed Products (other than to the extent AMT has the right to grant such rights under this Agreement) without TGC's prior written consent. If AMT fails or declines to assume the defense of any such claim or action within [\*\*] days after notice thereof, TGC may assume the defense of such claim or action for the account and at the risk of AMT, and any Liabilities related thereto shall be conclusively deemed a liability of AMT; provided however, that TGC shall not settle such claim or action if such settlement affects Licensed Patent Rights other than those specifically at issue in such claim or action without AMT's written permission, which shall not be unreasonably withheld. AMT shall pay promptly to the Indemnified Party any Liabilities to which the foregoing indemnity relates, as incurred. The indemnification rights of TGC or other Indemnified Party contained herein are in addition to all other rights which such Indemnified Party may have at law or in equity or otherwise.

## **16. INSURANCE**

16.1 AMT shall procure and maintain a policy or policies of commercial general liability insurance, including broad form and contractual liability, in a minimum amount of \$[\*\*] combined single limit per occurrence and in the aggregate as respects personal injury, bodily injury and property damage arising out of AMT's performance of this Agreement.

16.2 AMT shall, upon commencement of clinical trials involving Licensed Products, procure and maintain a policy or policies of product liability insurance in a minimum amount of

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\$[\*\*] combined single limit per occurrence and in the aggregate as respects bodily injury and property damage arising out of AMT's performance of this Agreement.

16.3 AMT shall provide TGC with certificates evidencing the insurance coverage required herein and all subsequent renewals thereof. Such certificates shall provide that AMT's insurance carrier(s) notify TGC in writing at least [\*\*] days prior to cancellation or material change in coverage. TGC will retain such certificates and notices from AMT's insurance carrier.

16.4 TGC shall periodically review the adequacy of the minimum limits of liability specified herein. Further, TGC reserves the right to require AMT to adjust such coverage limits in accordance with prevailing industry norms, to the extent TGC is required to do the same by UPenn pursuant to the UPenn Agreement. The specified minimum insurance amounts shall not constitute a limitation on AMT's obligation to indemnify TGC under this Agreement.

## **17. NOTICES**

17.1 Any notice or payment required to be given to either party shall be deemed to have been properly given and to be effective (a) on the date of delivery if delivered in person or (b) five (5) days after mailing if mailed by a nationally recognized courier or by first-class certified mail, postage paid, to the respective addresses given below, or to such other address designated by written notice, or sent via facsimile transmission to the number specified below.

For AMT:	Meibergdreef 61 P.O.Box 22506 1100 DA Amsterdam The Netherlands Fax: 31 (0) 20 566 92 72 Attention: CFO
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For TGC:	1100 Olive Way, Suite 100 Seattle, Washington 98101 Fax: +1 206 223-0288 Attention: Chief Executive Officer
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## **18. ASSIGNABILITY**

18.1 This Agreement is binding upon and shall inure to the benefit of the parties and their successors and assigns.

18.2 This Agreement may not be assigned by AMT without first obtaining the express written consent of TGC. TGC shall not unreasonably withhold consent for AMT to assign this Agreement to a Third Party in the event that AMT transfers all or substantially all of the business of AMT to which the License relates to a Third Party. Such Third Party shall assume all royalty obligations to TGC hereunder. TGC further acknowledges that it shall not be entitled to any Royalty or other compensation from the transaction pursuant to which the transfer to a Third Party of all or substantially all of the business of AMT to which the License relates took place.

## **19. WAIVER**

19.1 It is agreed that no waiver by either party hereto of any breach or default of any of the covenants or agreements herein set forth shall be deemed a waiver as to any subsequent and/or similar breach or default.

19.2 UPenn, by its signature on this Agreement, expressly acknowledges and agrees (1) to the terms of this sublicense; (2) to the extent that any term of this sublicense is in conflict with the UPenn Agreement, the terms of this Agreement shall govern as to the rights and obligations of AMT to TGC and AMT to UPenn pursuant to this Agreement; and (3) to the extent any term of this sublicense is in conflict with the UPenn Agreement, other than the UPenn Agreement terms of Section 4.1.2 and 4.1.5, 4.3.2 and 4.3.3, the terms of this Agreement shall govern as to the obligation of TGC to UPenn as they relate to this specific Agreement. TGC shall be obligated to comply with Sections 4.1.2, 4.1.5, 4.3.2 and 4.3.3 of the UPenn Agreement as they relate to the financial obligations of TGC to UPenn arising from the grant of this sublicense to AMT, unless mutually agreed otherwise between TGC and UPenn.

## **20. FAILURE TO PERFORM**

20.1 In the event of a failure of performance due under the terms of this Agreement and if it becomes necessary for either party to undertake legal action against the other on account thereof, then the prevailing party shall be entitled to reasonable attorney's fees in addition to costs and necessary disbursements.

## **21. GOVERNING LAW**

21.1 This Agreement shall be interpreted and construed in accordance with the laws of the Commonwealth of Pennsylvania without regard to the principles of conflicts of laws, but the scope and validity of any patent or patent application shall be governed by the applicable laws of the country of such patent or patent application. Each party hereby submits itself to the non-exclusive jurisdiction of the federal or state courts located in the Commonwealth of Pennsylvania, and any courts of appeal therefrom, and waives any objection (on grounds of lack of jurisdiction, or forum non conveniens or otherwise) to the exercise of such jurisdiction over it by any such courts, in connection with any action that may be brought in such courts.

## **22. FORCE MAJEURE**

22.1 For a period of ninety (90) days, the parties to this Agreement shall be excused from any performance required hereunder if such performance is rendered impossible or unfeasible due to any catastrophe or other major event beyond their reasonable control, including, without limitation, war, riot, and insurrection; laws, proclamations, edicts, ordinances or regulations; strikes, lockouts or other serious labor disputes; and floods, fires, explosions, or other natural disasters. When such events have abated, the parties' respective obligations hereunder shall resume.

## **23. RIGHTS IN BANKRUPTCY**

23.1 All rights and licenses (including but not limited to the License) granted under or pursuant to this Agreement by TGC to AMT are, and shall otherwise be deemed to be, for

purposes of Section 365(n) of the U.S. Bankruptcy Code, licenses of right to "intellectual property" as defined under Section 101 of the U.S. Bankruptcy Code. The parties agree that AMT, as licensee of such rights under this Agreement, shall retain and may fully exercise all of its rights and elections under the U.S. Bankruptcy Code and to the fullest extent permitted by law.

## **24. MISCELLANEOUS**

24.1 The headings of the several sections are inserted for convenience of reference only and are not intended to be a part of or to affect the meaning or interpretation of this Agreement. The English language is the official language of this Agreement and that text of the Agreement shall prevail over any translation thereof.

24.2 This Agreement will not be binding upon the parties until it has been signed below on behalf of each party, in which event, it shall be effective as of the dated recited on page one.

24.3 No amendment or modification hereof shall be valid or binding upon the parties unless made in writing and signed on behalf of each party.

24.4 This Agreement embodies the entire understanding of the parties and shall supersede all previous communications, representations or understandings, either oral or written, between the parties relating to the subject matter hereof.

24.5 If any provisions contained in this Agreement are or become invalid, are ruled illegal by any court of competent jurisdiction or are deemed unenforceable under then current applicable law from time to time in effect during the term hereof, it is the intention of the parties that the remainder of this Agreement shall not be affected thereby, provided that a party's rights under this Agreement are not materially affected. It is further the intention of the parties that in lieu of each such provision which is invalid, illegal, or unenforceable, there be substituted or added as part of this Agreement a provision which shall be as similar as possible in economic and business objectives as intended by the parties to such invalid, illegal or unenforceable, provision, but shall be valid, legal and enforceable.

IN WITNESS WHEREOF, both TGC and AMT have executed this Agreement, in duplicate originals, by their respective officers hereunto duly authorized, on the day and year hereinafter written.

**Amsterdam Molecular Therapeutics, B.V.**

**Targeted Genetics Corporation**

By /s/ Ronald HW. Lorijn  
Name Ronald H.W. Lorijn  
Title CEO

By /s/ H. Stewart Parker  
Name H. Stewart Parker  
Title President and CEO

Acknowledged and accepted by:

The Trustees of the University of Pennsylvania

By /s/ John S. Zawad, Ph.D.  
Name John S. Zawad, Ph.D.  
Title Managing Director

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**ATTACHMENT 1**

Articles from the UPenn Agreement which would be added to this Agreement, pursuant to Section 11.4 of this Agreement.

9.5- Penn hereby represents that (i) to its knowledge it has the lawful right to grant the licenses granted herein, and (ii) all actions necessary with respect to due authorization, execution and performance of this Agreement to make it legal, valid, binding and enforceable with regard to Penn have been taken.

12.1 Targeted shall comply with all prevailing laws, rules and regulations pertaining to the development, testing, manufacture, marketing, sale, use, import or export of products. Without limiting the foregoing, it is understood that this Agreement may be subject to United States laws and regulations controlling the export of technical data, computer software, laboratory prototypes and other commodities, articles and information, including the Arms Export Control Act as amended in the Export Administration Act of 1979, and that the parties' obligations hereunder are contingent upon compliance with applicable United States export laws and regulations. The transfer of certain technical data and commodities may require a license from the cognizant agency of the United States Government and/or written assurances by Targeted that Targeted shall not export data or commodities to certain foreign countries without prior approval of such agency. Penn neither represents that a license is not required nor that, if required, it will issue.

12.9 Nothing in this Agreement, express or implied, is intended to confer on any person, other than the parties hereto, the Covered Affiliates, or their permitted assigns, any benefits, rights or remedies.

12.10 Penn and Targeted shall not discriminate against any employee or applicant for employment because of race, color, sex, sexual or affectional preference, age, religion, national or ethnic origin, or handicap.

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EXECUTION

**UNIVERSITY of PENNSYLVANIA**

First Amendment to License Agreement

This First Amendment to the License Agreement (as defined below) effective as of June 28, 2013 (this "Amendment"), is made by and between AmpliPhi Biosciences, Inc., successor in interest to Targeted Genetics, Inc., having a principal place of business at 4870 Sadler Road, Suite 300, Glen Allen, VA 23060 ("AmpliPhi") and uniQure Biopharma B.V., formerly known as Amsterdam Molecular Therapeutics B.V., a Dutch limited liability company having a principal place of business at Meibergdreef 61, 1105 BA Amsterdam, The Netherlands ("uniQure") and amends the License Agreement dated December 5, 2006 by and between AmpliPhi and uniQure (the "Agreement" or "License Agreement").

**Recitals**

WHEREAS, AmpliPhi has exclusively licensed the Licensed Patent Rights (relating to an AAVI Vector gene delivery system) from the Trustees of the University of Pennsylvania ("Penn") as part of a license agreement entered into between AmpliPhi and Penn with the effective date of June 1, 2002 and as subsequently amended and restated ("Penn Agreement");

WHEREAS, uniQure has developed and is commercializing an AAVI product to treat LPL type 1 and LPL type 5 deficiencies that requires a license to the Licensed Patent Rights;

WHEREAS, AmpliPhi granted to uniQure a non-exclusive sublicense under the Licensed Patent Rights pursuant to the License Agreement, which does not permit further downstream sublicensing by uniQure; and

WHEREAS, uniQure desires to sub-sublicense certain of the Licensed Patent Rights to Chiesi Farmaceutici, S.p.A, an Italian Corporation ("Chiesi") under a certain Commercialization Agreement between uniQure and Chiesi dated April 29, 2013, a complete and accurate copy of which has been provided to AmpliPhi

and Penn, in order to more widely market and distribute the Licensed Products (the “Commercialization Agreement”).

NOW, THEREFORE, the parties hereto hereby agree as follows:

- 1) Unless otherwise defined herein, capitalized terms will have the meanings given them in the License Agreement. Section references are to the License Agreement, unless otherwise stated.
- 2) The purported Amendment No. 1 dated March 12, 2012 to the License Agreement entered into between AmpliPhi and uniQure was not executed by, agreed to, or consented to by Penn and therefore was not properly executed. For clarity, such Amendment No. 1 is hereby declared void in its entirety, ab initio, and is of no force or effect. All terms of the original License Agreement, including the terms purported to be modified by Amendment No. 1, remain in full force and effect, except as expressly modified by this Amendment.
- 3) Section 1.10 is hereby amended and restated in its entirety:

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1.10 “Net Sales” means (a) the actual gross invoice price of Licensed Products sold by uniQure, its Affiliates or its sublicensees to any Third Party, less, to the extent included therein, the total of (i) ordinary and customary trade discounts; (ii) sales and excise taxes, and other similar taxes, customs duty and compulsory payments to governmental authorities actually paid or deducted and related to the sale (excluding what is commonly known as income taxes); and (iii) credits given to customers for rejects or returns of the product. For purposes hereof, Net Sales shall not include sales of a Licensed Product from uniQure, an Affiliate of uniQure or a sublicensee to another of these entities; it being intended that Net Sales shall only include sales to Third Parties.

- 4) Section 1.13 is hereby amended and restated in its entirety:

1.13 “Third Party” means any Person, other than: (i) uniQure or its respective Affiliates, successors or assigns, (ii) AmpliPhi or its respective affiliates, successors or assigns, or (iii) sublicensees of uniQure, including Chiesi, or their respective affiliates, sublicensees, distributors, successors or assigns, or (iv) collaborators or distributors of uniQure or any of their respective affiliates, sublicensees, successors or assigns.

- 5) New Section 1.15 is hereby added to the License Agreement immediately following Section 1.14:

1.15 “Restricted Sublicensing Rights” means a non-exclusive sublicense under the Licensed Patent Rights from uniQure to sublicensees of uniQure, including Chiesi, pursuant to the sublicensing conditions contained in this Agreement.

- 6) Section 2.1 is hereby amended and restated in its entirety:

2.1 AmpliPhi grants to uniQure a non-exclusive license under the Licensed Patent Rights in each country of the world where there are Valid Claims of Licensed Patent Rights, to make, develop, use, sell offer to sell, and import Licensed Products, with Restricted Sublicensing Rights (the “License”).

- 7) New Section 2.4 is hereby added to the License Agreement immediately following Section 2.3:

2.4 The Restricted Sublicensing Rights conferred upon uniQure shall be subject to the following terms and conditions:

- a. uniQure must obtain the prior written consent of AmpliPhi and Penn to any sublicense or to any amendment modification or waiver thereto, which consent shall not be unreasonably withheld.
- b. AmpliPhi and Penn hereby consent to the Commercialization Agreement in effect as of the date hereof, provided that (a) in the event of a conflict between the terms of the Commercialization Agreement and this Agreement, this Agreement shall control and (b) for clarity, Chiesi agrees

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to comply with the obligations of sublicensees hereunder, including but not limited to the obligations under this Section 2.4 and Sections 4.6, 16.2, 17, and that Penn is a third party beneficiary under the Commercialization Agreement to the extent relevant for or applicable to the sublicenses hereunder.

- c. Any sublicense shall be subject to the terms and conditions granted to uniQure under this Agreement.
- d. No sublicensee shall have the power to grant further sublicenses without the express approval of AmpliPhi and consent of Penn, which approval and consent shall not be unreasonably withheld, except that Chiesi may further sublicense its rights to sub-distributors ( each a “sub-sublicensee”) as provided in the Commericalization Agreement on condition that any such downstream sublicense agreement (“sub-sublicense”) requires the sub-sublicensee to comply with the applicable terms of this Agreement and prohibits further sublicensing. For clarity, the sub-sublicensee shall be prohibited from further sublicensing. Except when used in Section 1.15 and this Section 2.4 d. the term sublicense includes any sub-sublicense and the term sublicensee includes any sub-sublicensee.
- e. uniQure shall forward to AmpliPhi and Penn, within [\*\*] days of execution, a complete and accurate copy written in the English language of any sublicense granted hereunder and any amendment, modification or waiver thereto. AmpliPhi’s and Penn’s receipt of such sublicense shall not constitute an approval of such sublicense or a waiver of any of Penn’s or AmpliPhi’s rights or uniQure’s obligations hereunder.
- f. Notwithstanding any such sublicense, uniQure shall remain primarily liable to AmpliPhi for all of uniQure’s duties and obligations contained in this Agreement, and any act or omission of an Affiliate or sublicensee of uniQure which would be a breach of this Agreement if performed by uniQure shall be deemed to be a breach by uniQure of this Agreement.

- 8) Section 4.1 is hereby amended and restated in its entirety:

4.1 uniQure shall pay to AmpliPhi earned royalties based on Net Sales ("Royalties") in the amount of: (i) [\*\*] percent ([\*\*]%) of Net Sales of Licensed Products if cumulative annual Net Sales are less than or equal to \$[\*\*] US Dollars), (ii) [\*\*] percent ([\*\*]%) of Net Sales of Licensed Products if cumulative annual Net Sales are greater than \$[\*\*] US Dollars) but less than or equal to \$[\*\*] US Dollars), (iii) [\*\*] percent ([\*\*]%) of Net Sales of Licensed Products if cumulative annual Net Sales are greater than \$[\*\*] US Dollars) but less than or equal to \$[\*\*] US Dollars), and (iv) [\*\*] percent ([\*\*]%) of Net Sales of Licensed Products if cumulative annual Net Sales are greater than \$[\*\*] US Dollars). The Net Sales thresholds in sections (i) - (iv) above will be applied separately to each distinct Licensed Product, but where the same Licensed Product is packaged or

labeled differently in different nations, or otherwise to accommodate the same for different markets or indications, the Net Sales thereof in all such nations, markets and indications shall be aggregated.

- 9) It is recognized and accepted by the parties that a Milestone as stated under Section 4.2.5 of the License Agreement was achieved on November 2, 2012 upon European regulatory approval for marketing authorization of Glybera indicated for treatment of LPL deficiency Type 1 (the "2012 Milestone"). It is also recognized by the parties that a Milestone Payment for the 2012 Milestone (the "2012 Milestone Payment") has not been made by uniQure to AmpliPhi. Pursuant to Section 4.1.8 of the Penn Agreement, Penn is entitled to receive [\*\*]% of said 2012 Milestone Payment from AmpliPhi, amounting to \$[\*\*] US Dollars). As a negotiated settlement, not being construed to alter or set precedent for this milestone or the resulting Milestone Payments as they relate to regulatory approvals for future indications, and as full and final settlement for any obligation to uniQure that might remain to pay the 2012 Milestone Payment, uniQure shall pay the amount of USD [\*\*] and AmpliPhi shall pay the amount of USD [\*\*] due to Penn within [\*\*] days after execution of this Amendment as a negotiated settlement for the 2012 Milestone Payment. Further, AmpliPhi and uniQure agree to negotiate in good faith over the next [\*\*] days concerning the license of additional rights from AmpliPhi, subject to existing license obligations and any consents required by Penn. Penn shall be entitled to be part of these discussions between AmpliPhi and uniQure.

- 10) Section 4.6 is hereby amended and restated in its entirety:

4.6 uniQure, its Affiliates and sublicensees must maintain, complete and accurate books and records which enable the Royalties, fees, and payments payable under this Agreement to be verified. The records for each calendar quarter must be maintained for [\*\*] years after the submission of each report under Article 4. Upon reasonable prior written notice to uniQure, uniQure must provide Penn and/or AmpliPhi with access to all of its books and records relating to the Sales of Licensed Products by uniQure, its Affiliates, and sublicensees in order to conduct a review or audit of those books and records. Access to these books and records pertaining to Net Sales must be made available no more than [\*\*] for each Licensed Product, during normal business hours, and [\*\*] for each Licensed Product during each of the [\*\*] years after expiration or termination of this Agreement. If a review or audit of the books of uniQure determines that any of uniQure, its Affiliates, or sublicensees has underpaid royalties on a Licensed Product by [\*\*] percent ([\*\*]%) or more, uniQure must pay the documented costs and expenses of Penn and/or AmpliPhi and their accountants in connection with such review or audit. Further, uniQure agrees to conduct, at Penn's expense (except in the case of an underpayment, as provided in the preceding sentence), an independent audit of Sales and Royalties with respect to a Licensed Product at least every [\*\*] years once annual Sales of such Licensed Product are greater than [\*\*] US Dollars (\$[\*\*]) per annum. The audit shall address, at a minimum, the amount of gross sales by or on behalf of uniQure, its Affiliates, or sublicensees

during the audit period, the amount of funds owed to uniQure under this Agreement, and whether the amount owed has been paid to uniQure and is reflected in the records of uniQure. A report by the auditors shall be submitted promptly to Penn and AmpliPhi upon completion.

- 11) Section 15.2 is hereby amended to require that any sublicensees indemnify Penn and its Trustees, officers, agents, and employees, both collectively and severally, to the same extent that uniQure indemnifies Penn under the current Section 15.2.
- 12) Section 16 is hereby amended to require that any sublicensees maintain insurance commensurate with the insurance that uniQure is required to maintain thereunder.
- 13) Section 17.1 is hereby amended and restated in its entirety as follows:

17.1 Any notice or payment required to be given to either party shall be deemed to have been properly given and to be effective (a) on the date of delivery if delivered in person or (b) five (5) days after mailing if mailed by a nationally recognized courier or by first-class certified mail, postage paid, to the respective addresses given below, or to such other address designated by written notice, or sent via facsimile transmission to the number specified below:

For uniQure:	Meibergdreef 61, 1105 BA Amsterdam The Netherlands Attn: Fax:
For AmpliPhi:	4870 Sadler Road, Suite 300 Glen Allen, VA 23060 Attn: Fax:

A copy of each notice shall be delivered to Penn as follows:



Center for Technology Transfer  
3160 Chestnut Street  
Suite 200  
Philadelphia, PA 19104  
Attn: Executive Director  
Fax: 215-898-9519

- 14) Section 24.3 is hereby amended and restated in its entirety as follows:

24.3 No amendment or modification hereof or waiver of obligations hereunder shall be valid or binding upon the parties unless made in writing and signed on behalf of each party, including, for clarity, Penn.

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- 15) This Amendment, together with the License Agreement, constitutes the entire agreement between the parties.

- 16) This Amendment may be executed in two or more counterparts, each of which shall be deemed an original and together shall be deemed one and the same instrument.

IN WITNESS WHEREOF, the parties, intending to be legally bound, have caused this Amendment to be executed by their duly authorized representatives.

AMPLIPHI BIOSCIENCES, INC.

By: /s/ Philip Young  
Name: Philip Young  
Title: CEO  
Date: 17, July 2013

UNIQUE BIOPHARMA B.V.

By: /s/ PJ Morgan  
Name: PJ Morgan  
Title: CFO  
Date: 15, July 2013

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Acknowledged and Agreed:

THE TRUSTEES OF THE UNIVERSITY OF PENNSYLVANIA

By: /s/ John S. Swartley  
Name: John S. Swartley, PhD  
Title: Executive Director, Center for Technology Transfer  
Date: June 28, 2013

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Confidential Materials omitted and filed separately with the Securities and Exchange Commission. Double asterisks denote omissions.

## EXCLUSIVE LICENSE AGREEMENT

THIS Exclusive License Agreement (“Agreement”), effective as of 07 July 2008 (“Effective Date”), is entered into by and between St. Jude Children’s Research Hospital, Inc., a Tennessee not-for-profit corporation located at 262 Danny Thomas Place, Memphis, Tennessee 38105 (“ST. JUDE”) and Amsterdam Molecular Therapeutics B.V., a closed limited liability company organized and existing under the laws of the Netherlands, with registered offices at Meibergdreef 61, 1105 BA Amsterdam, the Netherlands, (each a “Party” and together the “Parties”).

### RECITALS

WHEREAS, ST. JUDE is the owner by assignment from Dr. John Gray of his entire right, title and interest in the Patent Rights and in the inventions described and claimed therein; and

WHEREAS, the Patent Rights relate to mechanisms for improving the expression of Factor IX in gene therapy vectors, including, the use of a specific Factor IX polynucleotide coding sequence designed for optimal expression, and, the use of transcriptional regulatory regions minimized in size so that they can be used to express Factor IX, as well as any other gene of interest, in a size-constrained environment such as in a self complementary gene therapy vector system; and

WHEREAS, under a separate agreement being entered into between the Parties on the same date as this Exclusive License Agreement (“Sponsored Research Agreement”), AMT engages ST. JUDE to develop a gene therapy vector incorporating certain features of the invention described in the Patent Rights but combined with certain technologies controlled by AMT (the “Vector”); and

WHEREAS, in order to carry out the work anticipated under the Sponsored Research Agreement, ST. JUDE will require access to technologies owned by AMT and AMT is willing to grant this access under a separate agreement being entered into between the Parties on the same date as this Exclusive License Agreement (“AMT Technology License Agreement”); and

### SIGNATURE VERSION

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WHEREAS, the Vector will be further developed and commercialized by AMT and so AMT require a license under the Patent Rights; and

WHEREAS, AMT wishes to enter into an agreement with ST. JUDE to obtain a license under the Patent Rights and ST. JUDE is willing to grant such a license to AMT under the terms and conditions set forth in this Agreement.

NOW, THEREFORE, in consideration of the mutual promises contained herein and other good and valuable considerations, the Parties do hereby agree as follows:

#### 1. DEFINITIONS

- 1.1 “Affiliate” shall mean any company, partnership or other business entity which Controls, is Controlled by or is under common Control with either Party. For the purposes of this definition “Control” means any of the following: (i) the possession, directly or indirectly, of the power to direct the management or policies of an entity, whether through ownership of voting securities, by contract or otherwise; (ii) ownership of fifty percent (50%) or more of the voting securities entitled to vote for the election of directors in the case of a corporation, or of fifty percent (50%) or more of the equity interest in the case of any other type of legal entity; (iii) status as a general partner in any partnership, or any other arrangement whereby a Party controls or has the right to control the board of directors or equivalent governing body of a corporation or other entity.
- 1.2 “AMT” shall mean Amsterdam Molecular Therapeutics B.V. and any Affiliates of Amsterdam Molecular Therapeutics B.V.
- 1.3 “Confidential Information” shall mean any confidential or proprietary non-public information furnished by one Party (the “Disclosing Party”) to the other Party (the “Receiving Party”) in connection with this Agreement.

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- 1.4 “Commercialise”, “Commercialisation” or “Commercialising” shall mean all activities relating to the import, advertising, promotion and other marketing, pricing and reimbursement, detailing, distribution, storage, handling, offering for sale and selling, customer service and support or post regulatory approval regulatory activities in relation to Licensed Product.
- 1.5 “Commercially Reasonable Efforts” shall mean efforts and resources commonly used by biotechnology companies of a similar size to AMT based on funds raised to Develop and Commercialise a product owned by such a company or to which it has rights, which product is at a similar stage in its development or product life and is of similar market potential to the Licensed Product in question and taking into account the patent and other proprietary position of the product.
- 1.6 “Development (and the corresponding verb “to Develop”)” shall mean all pre-regulatory approval development and regulatory activities regarding a product in a country conducted with the aim of obtaining such regulatory approval including:
  - (a) studies on the toxicological, pharmacological, metabolic or clinical aspects of a product conducted internally or by individual investigators or consultants; and

- (b) process development, process improvement, scale-up and recovery, the manufacture of clinical supplies of product, including failed batches, manufacture of qualification lots; and
- (c) preparing, submitting, reviewing or developing data or information for the purpose of submission to a regulatory authority to obtain, maintain and/or expand manufacturing and/or regulatory approval of a product including data management, statistical designs and studies, document preparation, and other administration; and

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- (d) all post regulatory approval regulatory activities and adverse event reporting in relation to products.

1.7 “Field” shall mean gene therapy for the therapy or prophylaxis of Haemophilia B.

1.8 “Investigational New Drug Application (“INDA”)” shall mean an application submitted to the FDA or a foreign equivalent requesting permission to conduct human clinical studies with an investigational new drug or to conduct human clinical studies with an existing drug for a new use.

1.9 “Licensed Product” shall mean all products containing Factor IX that are covered by Valid Claims of the Patent Rights to any extent.

1.10 “Net Sales” shall mean the sum of all amounts actually invoiced by AMT (or Sublicensees as appropriate) from persons or entities for sales of Licensed Products, less the following deductions and offsets, to the extent such sums are actually incurred, paid or credited by AMT (or Sublicensees as appropriate) and not otherwise reimbursed:

- (a) normal and customary trade, cash and quantity discounts actually given, credits, price adjustments or allowances for damaged products, returns or rejections of products;
- (b) chargeback payments and rebates (or the equivalent thereof) for product granted on a customary trade basis to group purchasing organisations, managed health care organisations or to federal, state/provincial, local and other governments, including their agencies, or to trade customers;
- (c) reasonable and customary freight, shipping insurance and other transportation expenses directly related to the sale of product (if actually borne by AMT or Sublicensees without reimbursement from any third party);

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- (d) required distribution commissions/fees payable to any third party providing distribution services to AMT or Sublicensees;

- (e) sales, value-added, excise taxes, tariffs and duties, and other taxes and government charges directly related to the sale, to the extent that such items are included in the gross invoice price of product and are actually borne by AMT or its Sublicensees without reimbursement from any third party (but not including taxes assessed against the income derived from such sale); and

- (f) actual uncollectible amounts for product where collectibility is determined in accordance with IFRS consistently applied to all AMT products.

Sales of products intended for resale to third parties, and made internally amongst AMT and Sublicensees shall not be deemed sales for purposes of calculating “Net Sales” (however, sales to a third party other than a Sublicensee or Affiliate shall be included in the calculation of “Net Sales”).

1.11 “Patent Rights” shall mean (i) U.S. Provisional Patent Application No. 60/612,135 filed September 22, 2004 entitled “Improved Expression of Factor IX in Gene Therapy Vectors” (ST. JUDE reference no. SJ-04-0024); (ii) all patent applications filed claiming priority from the above including all provisional patent applications and all national, regional and international patents and patent applications; (iii) all patent applications filed claiming priority from any of the above including any divisional, continuation, or continuation-in-part; (iv) any patent issued on any of the foregoing; (v) any reissue or extension of such patent; and (vi) any foreign counterparts to such patents and patent applications and applications and/or patents or the equivalent thereof claiming priority to or from any of the above.

1.12 “Phase I Clinical Trial” shall mean a human clinical trial, the principal purpose of which is a preliminary determination of safety in healthy individuals or patients as required in 21

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C.F.R. §312, or a similar clinical study prescribed by the regulatory authorities in a country other than the United States.

1.13 “Phase II Clinical Trial” shall mean (i) a human clinical trial, for which a primary endpoint is a preliminary determination of efficacy or dose ranges in patients with the disease target being studied as required in 21 C.F.R. §312.21(b), as maybe amended from time to time, or a similar clinical study prescribed by the regulatory authorities in a country other than the United States, or (ii) a combined Phase II and Phase III Clinical Trial which enrolls at least forty (40) patients, or any Phase III Clinical Trial performed in lieu of a Phase II study.

1.14 “Phase III Clinical Trial” shall mean a human clinical trial, the principal purpose of which is to establish safety and efficacy in patients with the disease target being studied as required in 21 C.F.R. §312, or similar clinical study prescribed by the regulatory authorities in a country other than the United States. A Phase III Clinical Trial shall also include any other human clinical trial intended as a pivotal study, whether or not such study is a traditional Phase III study.

1.15 “Royalty Reporting Period” shall mean the partial calendar half year commencing on the date on which a Licensed Product is first sold and every complete or partial calendar half year thereafter during which royalty payment obligations under this Agreement remain in effect.

1.16 “Sublicensee” shall mean any sublicensee of the rights granted to AMT under this Agreement, as further described in Section 2.1(i).

- 1.17 “**ST. JUDE Improvements**” shall mean any improvement or enhancement (whether patentable or not) to the inventions of a Valid Claim of the Patent Rights in the Field that is discovered, developed or otherwise acquired by ST. JUDE after the Effective Date

pursuant to Section 2.1(v) (but, for clarity, outside the scope of the Sponsored Research Agreement) and in relation to which a patent application is filed by ST. JUDE.

- 1.18 “**Valid Claim**” shall mean a claim of an issued and unexpired patent or pending published patent application included within Patent Rights, which has not been held permanently revoked, unenforceable or invalid by a decision of a court or other governmental agency of competent jurisdiction, unappealable or un-appealed within the time allowed for appeal, and which has not been admitted to be invalid or unenforceable through reissue or disclaimer or otherwise.

## 2. GRANT

### 2.1 License Grant

- (i) ST. JUDE hereby grants to AMT and AMT accepts, subject to the terms and conditions herein, an exclusive worldwide license under the Patent Rights to research, have researched, Develop, have Developed, make, have made, import, distribute, use and Commercialise Licensed Products in the Field. Such license shall include the right to grant sublicenses provided that AMT shall remain responsible for compliance by Sublicensees with the terms and conditions of this Agreement. Within [\*\*] days of the grant of each sublicense under this Agreement, AMT shall inform ST. JUDE in writing of the identity of the Sublicensee and provide a copy of the sublicense agreement but showing only those terms directly pertaining to the sublicense itself, with all other terms including financial terms redacted.
- (ii) The license granted herein is subject to the rights, conditions and limitations imposed by U.S. law on inventions and discoveries conceived or first actually reduced to practice during the course of research funded by a U.S. federal agency that are relevant to the Patent Rights. The words “exclusive license” as used herein

shall mean exclusive except for the royalty-free non-exclusive license granted to the U.S. government by ST. JUDE pursuant to 35 USC Section 202 (c) (4) for any Patent Rights claiming any invention subject to such license as defined under 35 USC Section 201 and any other federal laws and applicable regulations.

- (iii) To the extent that Licensed Products embody Patent Rights conceived or first actually reduced to practice during the course of research funded by a U.S. federal agency, AMT agrees that such Licensed Products shall be manufactured substantially in the United States in accordance with 35 U.S.C. Section 204.
- (iv) Title to the Patent Rights shall remain with ST. JUDE and ST. JUDE retains the right to license the Patent Rights to other entities outside the Field and to use the Patent Rights for internal and collaborative research outside the Field.
- (v) The license granted under Section 2.1(i) of this Agreement is subject to the non-transferable right of ST. JUDE under the Patent Rights solely to perform internal and collaborative research and education in the Field with academic collaborators. To the extent that such research involves pre-clinical or clinical research the data and other results of such research, including any IND package and a copy of any interim or final clinical research report shall be made available by ST. JUDE to AMT and AMT shall be permitted to utilise the same only for lawful purposes in its dealings with the FDA. ST. JUDE will at its own cost procure that this is possible under the terms of any agreement between it and such academic collaborations.
- 2.2 **ST. JUDE Improvements.** If any ST. JUDE Improvements are made by ST. JUDE during the term of this Agreement, AMT shall have the first right of refusal to such ST. JUDE Improvements. ST. JUDE will disclose any such ST. JUDE Improvement to AMT by notice in writing. AMT shall treat any such disclosure as Confidential Information and

shall only use such disclosure to consider its licensing interest. AMT shall have [\*\*] days from the date of such disclosure in which to indicate to ST. JUDE by notice in writing whether it wishes to negotiate a license with grant terms the same as those set out in Section 2.1. If AMT does so indicate that it wishes to take a license within the notice period, the Parties shall negotiate in good faith the financial and other terms of such license during the subsequent period of [\*\*] days. If the Parties cannot reach agreement during the negotiation period, ST. JUDE will be free to deal with third parties in respect of the said ST. JUDE Improvements in the Field. During the period from first notification by ST. JUDE of the ST. JUDE Improvement to AMT until the expiration of the aforesaid [\*\*] day period, the ST. JUDE shall not enter into arrangements or agreements with any third party concerning the ST. JUDE Improvements in the Field.

## 3. DILIGENCE OBLIGATIONS AND ANNUAL PROGRESS REPORT

- 3.1 **Use of Commercially Reasonable Efforts.** With effect from completion or termination of the Research Program under the Sponsored Research Agreement (“Commencement Date”) AMT shall use Commercially Reasonable Efforts to diligently Develop and Commercialize Licensed Products whether by itself or through its Sublicensee(s).
- 3.2 **Annual Progress Reports.** Within [\*\*] days after each anniversary of the Commencement Date, AMT shall furnish ST. JUDE with a written report summarizing efforts and achievements toward Developing and Commercializing Licensed Products, including the status of any regulatory submissions,

clinical trials and sublicensing activities. This report shall also include a statement regarding insurance coverage in accordance with Section 8.1 (iii) below.

#### 4. PAYMENTS

- 4.1 License Fee. In partial consideration of the rights granted to AMT under this Agreement and to reimburse ST. JUDE for patent expenses already incurred in pursuing the Patent

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Rights, AMT shall pay [\*\*] U.S. dollars to ST. JUDE within [\*\*] days of the full execution of this Agreement. This license fee payment is nonrefundable and not creditable against any other payments due to ST. JUDE under this Agreement.

- 4.2 Annual Maintenance Fee. AMT shall pay ST. JUDE an annual fee of [\*\*] U.S. dollars within [\*\*] days of January 1. This fee shall be creditable to royalties and milestones which are due in the same calendar year.

- 4.3 Milestone Payments. AMT shall pay ST. JUDE the following milestone payments on the first occurrence of the following milestone events:

- (i) [\*\*] U.S. dollars upon [\*\*];
- (ii) [\*\*] U.S. dollars upon [\*\*]; and
- (iii) [\*\*] U.S. dollars upon [\*\*].

Each of the milestone payments the subject of this Section shall only be payable by AMT upon the first occurrence of the applicable event whenever it occurs.

- 4.4 Royalties. AMT shall pay ST. JUDE [\*\*] percent ([\*\*]%) of Net Sales of Licensed Products sold by AMT itself or Sublicensees on a country by country basis until expiry of the Valid Claims of the Patent Rights in the country of sale that cover the product and render it a Licensed Product.

- 4.5 Sublicense Consideration. In addition to the royalty obligation as set forth under Section 4.4, AMT shall pay to ST. JUDE the following percentages of consideration received for sublicenses under this Agreement:

- (i) [\*\*] percent ([\*\*]%) for a sublicense granted [\*\*];
- (ii) [\*\*] percent ([\*\*]%) for a sublicense granted [\*\*]; and

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- (iii) [\*\*] percent ([\*\*]%) for a sublicense granted [\*\*].

This payment shall be due, without the need for invoice from ST. JUDE, within [\*\*] days of the receipt the payment made to AMT by a Sublicensee under a sublicense agreement. Such sublicense consideration shall include consideration of any kind received by AMT from a Sublicensee for the grant of a sublicense under this Agreement, such as upfront fees or milestone fees and including any premium paid by the Sublicensee over the Fair Market Value (as such term is defined in subsection (iii) below) for stock of AMT in consideration for such sublicense. However, not included in such sublicense consideration are:

- (i) Support for research, Development and/or manufacturing activities corresponding directly to the Development of Licensed Products, which do not exceed the fully-burdened cost for undertaking such research, Development, and/or manufacturing performed by or for AMT (including third parties on AMT's behalf), each pursuant to a specific agreement including a performance plan and commensurate budget;
- (ii) Proceeds derived from debt financing, to the extent that such financing is at market rates, and any loans to AMT by the Sublicensee;
- (iii) Consideration received for the purchase of an equity interest in AMT to the extent that the price per share for such equity does not exceed by more than [\*\*] percent ([\*\*]%) the Fair Market Value of AMT's stock. The term Fair Market Value shall mean the average price that the stock in question is publicly trading at for twenty (20) days prior to the announcement of its purchase by the Sublicensee or if the stock is not publicly traded, the value of such stock as determined by the most recent private financing through a financial investor (an entity whose sole interest in AMT is financial);
- (iv) Reimbursement of AMT's patent costs related to Patent Rights; and

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- (v) Any and all amounts paid to AMT by a Sublicensee as royalties on sales of Licensed Product sold by the Sublicensee under a sublicense.

- 4.6 Late Payment. For any late payment AMT shall pay an interest penalty based on the amount owed at a daily accrual rate equal to the lesser of [\*\*] percent ([\*\*]%) per annum or the highest rate permissible by law.

#### 5. ROYALTY REPORTS; PAYMENTS; RECORDS

- 5.1 First Sale. AMT shall report to ST. JUDE the date of first commercial sale of a Licensed Product within [\*\*] days of its occurrence.

5.2 **Reports and Payments.** Within [\*\*] days after the conclusion of each Royalty Reporting Period, AMT shall deliver to ST. JUDE a report of Net Sales for each Licensed Product during the prior Royalty Reporting Period on a country-by-country basis. Such report shall include the amount of gross sales and the amount of all deductions and reductions taken in each category identified in the definition of Net Sales in sufficient detail to allow ST. JUDE to verify the Net Sales calculation, the amount of Net Sales, and the total royalty payable on Net Sales in U.S. dollars, together with the exchange rates used for conversion. All such reports shall be considered Confidential Information of which AMT is the Disclosing Party and the provisions of Section 7 of this Agreement shall apply to such reports. If no royalties are due to ST. JUDE for any Royalty Reporting Period, the report shall so state. Concurrent with the report, AMT shall remit to ST. JUDE any payment due for the applicable Royalty Reporting Period. Unless other arrangements are made, payment shall be remitted to the following address:

St. Jude Children's Research Hospital  
P.O. Box 1000, Department # 516  
Memphis, TN 38148-0516

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5.3 **Records.**

- (i) AMT shall maintain, and shall require its Sublicensees to maintain complete and accurate records of all Net Sales under this Agreement which records shall contain sufficient information to permit auditors to confirm the accuracy of any reports delivered to ST. JUDE under Section 5.2. The relevant party shall retain such records relating to a given Royalty Reporting Period for at least [\*\*] years after the conclusion of that Royalty Reporting Period.
- (ii) Upon [\*\*] working days notice by ST. JUDE, ST. JUDE shall have the right, at its expense, to cause accountants from a nationally-recognized accounting firm to inspect the records of AMT (but not Sublicensees) for the period covering Royalty Reporting Periods ending no more than [\*\*] years prior to the date of the inspection (including records of royalty accounting received from their Sublicensees) during normal business hours for the sole purpose of verifying any reports and payments delivered under this Section 5.2. ST. JUDE may exercise its rights under this Section 5.3 only [\*\*].
- (iii) The Parties shall reconcile any underpayment or overpayment within [\*\*] days after the auditor delivers the results of the audit. In the event that any audit performed under this Section reveals an underpayment in excess of [\*\*] percent ([\*\*]%) in any Royalty Reporting Period, AMT shall bear the full cost of such audit.
- (iv) Prior to any such audit taking place, such firm of accountants shall undertake to AMT that they shall keep all information and data contained in the records of AMT strictly confidential and shall not disclose such information or copies of such records to any third person including ST. JUDE, but shall only use the same for the

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purpose of the reviews and/or calculations which they need to perform in order to verify the reports delivered under Section 5.2 of this Agreement.

- (v) Upon timely request by ST. JUDE, AMT shall, at the expense of ST. JUDE, have any Sublicensee accounting to AMT for royalties audited under the audit provisions agreed between AMT and the Sublicensee, and AMT shall report to ST. JUDE the outcome. If there is a discrepancy identified upon such an audit the provisions of Section 5.3(iii) shall apply in like manner.

6. **PATENTS AND INFRINGEMENT**

6.1 **Responsibility for Patent Rights.** Title to all Patent Rights shall remain with ST. JUDE and ST. JUDE shall retain primary responsibility for the drafting, filing, prosecution, and maintenance of all Patent Rights. ST. JUDE shall appoint and retain external patent counsel approved by AMT. ST. JUDE shall keep AMT informed of all developments including promptly furnishing AMT with all patent office communications. ST. JUDE shall, to the satisfaction of AMT, implement all reasonable requests of AMT with respect to the drafting, filing, prosecution, and maintenance of all Patent Rights.

6.2 **Reimbursement of Patent Expenses.** AMT shall reimburse ST. JUDE for all out of pocket patent-related expenses incurred by ST. JUDE pursuant to Section 6.1 related to Patent Rights during the term of this Agreement within [\*\*] days after ST. JUDE invoices AMT subject to the provisions of this Section. Such invoice shall contain a breakdown of the expenses and be accompanied by supporting evidence of such expenses as appropriate including copies of invoices from external patent counsel. ST. JUDE shall instruct its patent counsel to communicate with AMT directly on all matters pertaining to the activities of patent counsel, including the giving of forward cost estimates, but copying ST. JUDE on all e-mails and other correspondence.

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6.2.1 If ST. JUDE grants an exclusive license under the Patent Rights outside of the Field, ST. JUDE shall promptly notify AMT. In such an event, from the effective date of that license, AMT shall be responsible for [\*\*] percent ([\*\*]%) of the patent-related expenses only.

6.3 **Abandonment.** AMT may elect, upon [\*\*] days written notice to ST. JUDE, to cease payment of the expenses associated with obtaining or maintaining patent protection for one or more patents or applications within the Patent Rights in one or more countries. In such event, AMT shall forfeit all rights under this Agreement with respect to such patent within the Patent Rights in such country(ies). ST. JUDE shall have the right, at its sole expense, to prepare, file, prosecute, and maintain any patents or patent applications under Patent Rights abandoned by AMT.

6.4 **Infringement.**

- (i) **Notification of Infringement.** Each Party agrees to provide written notice to the other Party promptly (i) after becoming aware of or having a reasonable suspicion of any infringement of the Patent Rights in the Field or (ii) upon becoming aware of any action alleging invalidity or non-

infringement of the Patent Rights in the Field.

- (ii) AMT Right to Enforce Patent Rights in the Field. AMT shall have the right, under its own control and at its own expense, to prosecute any third party infringement of the Patent Rights in the Field including negotiating sublicense agreements with such third parties at its discretion. At least [\*\*] days prior to a potential claim, AMT shall notify ST. JUDE in writing of the nature of the anticipated action and the party(ies) against whom the anticipated action may be taken. It is understood that any sublicense rights granted hereunder shall not forgive any royalty payments that would otherwise be due to ST. JUDE based on sales of Licensed Products by the Sublicensee without consultation with ST. JUDE. If AMT succeeds in any such

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infringement proceedings whether at trial or by way of settlement, any recovery by AMT in such proceedings brought by AMT shall first be used to reimburse AMT for all reasonable out-of-pocket costs and legal fees incurred to conduct such proceedings. Out of any remaining damages actually received by AMT relating to infringement of the Patent Rights, AMT shall pay to ST. JUDE an amount equivalent to the payment which would have been due to ST. JUDE on the balance as if they were Net Sales, along with an accounting of the recovery and the reasonable out-of-pocket costs and legal fees.

In the event that AMT fails to initiate an infringement action within [\*\*] months after it first becomes aware of such infringement or notifies ST. JUDE that it does not intend to initiate such action, ST. JUDE shall have the right to prosecute such infringement, under its sole control and its sole expense, and any recovery obtained shall be retained by ST. JUDE. AMT shall provide all necessary assistance to ST. JUDE in relation to such proceedings and ST. JUDE shall on demand by AMT indemnify AMT against the costs of such activity, unless AMT elects to be separately represented (which shall be at AMT's discretion), in which case such separate representation shall be at AMT's cost and expense.

- (iii) ST. JUDE as Indispensable Party. If, in the reasonable opinion of AMT's counsel, ST. JUDE should be a named party to any such suit, AMT may name ST. JUDE as a party, provided that AMT shall hold ST. JUDE harmless from, and if necessary indemnify ST. JUDE against, any costs (including attorney fees), expenses or liability that ST. JUDE may incur in connection with such action unless ST. JUDE elects to be separately represented in which case such separate representation shall be at ST. JUDE's own cost and expense.
- (iv) Cooperation. Each Party agrees to cooperate fully in any action under this Section 6.4, which is controlled by the other Party, provided that the controlling Party

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reimburses the cooperating Party promptly for any costs and expenses incurred by the cooperating Party in connection with providing such assistance.

- 6.5 Third Party Patent Rights. Each Party shall promptly notify the other Party of any third party patent rights relevant to the Development or Commercialisation of the Licensed Product of which it becomes aware including by making appropriate searches for these as and when it considers appropriate. To the extent possible, whilst preserving attorney-client privilege, the Parties' patent counsel shall share copies of all external and internal opinions on the likelihood of grant and/or validity of relevant third party patent rights

## 7. **CONFIDENTIAL INFORMATION; PUBLICITY**

### 7.1 Confidential Information.

- (i) Obligations. Except to the extent authorized in Section 7.1(i) of this Agreement, and for so long as the exceptions set out below in Section 7.1(ii) do not apply, the Receiving Party shall, in relation to any Confidential Information (i) maintain such Confidential Information in confidence using the same duty of care it would use to protect its own information of a like kind (and in any event no less than reasonable care), except that the Receiving Party may disclose or permit the disclosure of any Confidential Information to its Sublicensees (to the extent necessary to effect the relevant Sublicense) and its trustees, directors, officers, employees, consultants, and advisors who are obligated to maintain the confidential nature of such Confidential Information and who need to know such Confidential Information for the purpose of this Agreement and for other purposes that may be required or necessary pursuant to this Agreement such as communication with collaborators or regulatory agencies; (ii) use such Confidential Information solely for the purposes of this Agreement; and (iii) allow its Sublicensees, trustees or directors, officers, employees, consultants, and advisors to reproduce the Confidential Information

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only to the extent necessary for the purposes of this Agreement, with all such reproductions being considered Confidential Information.

- (ii) Exceptions. The obligations of the Receiving Party under Section 7.1(i) above shall not apply to the extent that the Receiving Party can demonstrate by written evidence that certain Confidential Information (a) was in the public domain prior to the time of its disclosure under this Agreement; (b) entered the public domain after the time of its disclosure under the Agreement through means other than an unauthorized disclosure resulting from an act or omission by the Receiving Party; (c) was independently developed or discovered by the Receiving Party without use of the Confidential Information; (d) is or was disclosed to the Receiving Party at any time, whether prior to or after the time of its disclosure under this Agreement, by a third party having no fiduciary relationship with the Disclosing Party and having no obligation to confidentiality with respect to such Confidential Information; or (e) was previously known to the Receiving Party from sources other than the Disclosing Party at the time of disclosure under this Agreement other than under an obligation of confidentiality.
- (iii) Allowed Disclosure. Notwithstanding the above obligations of confidentiality and non-use a Receiving Party may:
- (a) disclose Confidential Information to a competent authority as reasonably necessary to obtain regulatory approval in a particular jurisdiction to the extent consistent with the licenses granted under terms of this Agreement; and

- (b) disclose Confidential Information: (i) to the extent such disclosure is reasonably necessary to comply with the order of a court; or (ii) to the extent such disclosure is required to comply with a legal requirement,

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including to the extent such disclosure is required in publicly filed financial statements or other public statements under rules governing a stock exchange (e.g., Euronext, the rules of the United States Securities and Exchange Commission, NASDAQ, NYSE, or any other stock exchange on which securities issued by either Party may be listed); provided, to the extent possible bearing in mind such legal requirements and subject to the next sentence of this Section, such Party shall provide the other Party with a copy of the proposed text of such statements or disclosure [\*\*] Business Days in advance of the date on which the disclosure is to be made to enable the other Party to review and provide comments, unless a shorter review time is agreed. If the compliance with a legal requirement requires filing of this Agreement, the filing Party shall to the extent possible seek confidential treatment of portions of this Agreement from the relevant competent authority and shall provide the other Party with a copy of the proposed filings at least [\*\*] Business Days prior to filing for the other Party to review any such proposed filing. Each Party agrees that it will obtain its own legal advice with regard to its compliance with legal requirements and will not rely on any statements made by the other Party relating to such legal requirements; and

- (c) disclose Confidential Information by filing or prosecuting the Patent Rights, the filing or prosecution of which is contemplated by this Agreement, without violating the above confidentiality provisions; it being understood that publication of such filings occurs in some jurisdictions within [\*\*] months of filing; and
- (d) in the case where AMT is the Receiving Party disclose Confidential Information to AMT's contractors (including clinical researchers) distributors, sublicensee's, agents, consultants, as such Receiving Party

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reasonably determines is necessary to receive the benefit of the licenses and rights granted or available to it under this Agreement or to fulfil its obligations pursuant to this Agreement; provided, however, any such persons must be obligated to substantially the same extent as set forth in this Section to hold in confidence and not make use of such Confidential Information for any purpose other than that permitted by this Agreement; and

- (e) disclose Confidential Information: (i) to its actual or potential investment bankers; (ii) to existing and potential investors in connection with an offering or placement of securities for purposes of obtaining financing for its business and to actual and prospective lenders for the purpose of obtaining financing for its business; and (iii) to a bona fide potential acquirer or merger partner for the purposes of evaluating entering into a merger or acquisition, provided, however, any such persons must be obligated to substantially the same extent as set forth in this Section to hold in confidence and not make use of such Confidential Information for any purpose other than those permitted by this Agreement; and
- (f) disclose Confidential Information to its legal advisers for the purpose of seeking advice.

## 7.2 Use of Names.

- (i) AMT and its sublicensees shall not use the name "St. Jude Children's Research Hospital" or any variation of that name, or any trademarks or logos belong to ST. JUDE, or the names of any of ST. JUDE's trustees, officers, faculty, students, employees, or agents, or any adaptation of such names, or any term of this Agreement in any promotional material or other public announcement or disclosure

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or in connection with the marketing or sale of any Licensed Product without the prior written approval of ST. JUDE; except: (a) in annual reports or as part of required regulatory or financial disclosures to the FDA, Securities Exchange Commission or other federal or foreign agencies; and (b) where otherwise required by law, provided that AMT shall notify ST. JUDE in advance of any disclosure to be made under these exceptions.

- (ii) ST. JUDE shall not use the name "Amsterdam Molecular Therapeutics B.V." or any variation of that name, or any term of this Agreement in any promotional material or other public announcement or disclosure without the prior written approval of AMT; except: (a) as part of required reports to state or federal government entities; and (b) where otherwise required by law, provided that ST. JUDE shall notify AMT in advance of any disclosure to be made under these exceptions.

7.3 **Publication.** If ST. JUDE wishes to publish or otherwise publically disclose results obtained from its internal research under the Patent Rights in the Field, ST. JUDE shall submit to AMT a confidential copy of the intended publication or disclosure at least [\*\*] days prior to the proposed publication or other disclosure date. If, AMT believes that the publication or intended disclosure contains patentable subject matter or contains Confidential Information of AMT, AMT shall notify ST. JUDE in writing.

7.4 **Marking.** AMT shall mark all Licensed Products intended for use under Patent Rights with appropriate information with respect to Patent Rights in accordance with the statutes of the United States relating to the marking of patented articles (see 35 U.S.C. §287(a)) and corresponding foreign rules and regulations.

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## 8. INDEMNIFICATION AND INSURANCE



(i) Indemnity.

- (a) Except with respect to third party claims the subject of this Section, neither Party shall be liable to the other in contract, tort, negligence, breach of statutory duty or otherwise for any loss, damage, costs or expenses of any nature whatsoever incurred or suffered by the other or its Affiliates of a direct nature where the same is a loss of turnover, profits, business or goodwill; or an indirect or consequential or punitive nature, including any indirect or consequential economic loss or other indirect or consequential loss of turnover, profits, loss of enterprise value, business or goodwill or otherwise.
- (b) AMT shall indemnify, defend and hold ST. JUDE, the American Lebanese Syrian Associated Charities, Inc. (ALSAC; a non-profit, 501(c)(3) corporation which supports ST. JUDE), their present and former trustees, directors, governors, officers, agents, faculty, employees and students ("the Indemnitees") harmless as against any claims, demands, damages, judgments, fees (including reasonable attorneys fees), expenses, or other costs arising from or incidental to any product liability or other lawsuit, claim, demand or other action brought by a third party as a consequence of the use of clinical data provided by ST. JUDE, the practice of the Patent Rights or the sale of Licensed Products by AMT or Sublicensees, whether or not ST. JUDE, either jointly or severally, is named as a party defendant in any such lawsuit and whether or not ST. JUDE is alleged to be negligent or otherwise responsible for any injuries to persons or property. Such indemnity shall not extend to any claims, demands, damages, judgments, fees (including reasonable attorneys fees), expenses, or other costs to the extent that the same are determined to be the result of the willful misconduct of ST. JUDE, the American Lebanese Syrian Associated

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Charities, Inc., their present and former trustees, directors, governors, officers, agents, faculty, employees or students Practice of the Patent Rights or sale of Licensed Products by an Affiliate of AMT or an agent or a Sublicensee or a third party on behalf of or for the account of AMT or by a third party who purchases Licensed Product(s) from AMT, shall be considered AMT's practice of said Patent Rights for purposes of this Section. The obligation of AMT to defend, indemnify and hold harmless as set out in this Section shall survive the termination of this Agreement, shall continue even after assignment of rights and responsibilities to an Affiliate or Sublicensee, and shall not be limited by any other limitation of liability elsewhere in this Agreement.

- (c) In the event that it is ultimately determined that AMT is not obligated to indemnify, defend and hold harmless the Indemnitees as against any claims, demands, damages, judgments, fees (including reasonable attorneys fees), expenses, or other costs, the Indemnitees shall reimburse AMT for any and all costs and expenses (including lawyers' fees) incurred by AMT in its defense with respect to the Indemnitees.
- (ii) Procedures. The Indemnitees agree to provide AMT with prompt written notice of any claim, suit, action, demand, or judgment for which indemnification is sought under this Agreement. In no event shall AMT be liable for any claims, demands, damages, judgments, fees (including reasonable attorney's fees), expenses, or other costs that result from a delay by the Indemnitees in providing AMT with such notice. AMT agrees, at its own expense, to provide attorneys reasonably acceptable to Indemnitees to defend against any such claim, unless Indemnitees elect to be separately represented (which shall be at Indemnitee's discretion), in which event any costs incurred by the Indemnitees in relation to retaining their own attorneys shall be the sole responsibility of the Indemnitees. The Indemnitees shall

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cooperate fully with AMT in such defense and will permit AMT to conduct and control such defense and the disposition of such claim, suit, or action (including all decisions relative to litigation, appeal, and settlement). AMT agrees to keep ST. JUDE informed of the progress in the defense and disposition of such claim and to consult with ST. JUDE with regard to any proposed settlement.

- (iii) Insurance. Prior to initial human testing or sale of any Licensed Products and thereafter so long as Licensed Products are being sold in any particular country AMT shall establish and maintain appropriate insurance coverage in the minimum amount of [\*\*] dollars (\$[\*\*]) per claim, with an aggregate of [\*\*] dollars (\$[\*\*]), to cover any liability arising from AMT's indemnification obligations under this Section 8 with respect to such human testing or sale of Licensed Product. Prior to initial human testing or sale of any Licensed Product and thereafter so long as Licensed Products are being sold in any particular country, AMT shall establish and maintain, in each country in which AMT or Sublicensees shall test or sell Licensed Products, product liability or other appropriate insurance coverage in the minimum amount of [\*\*] dollars (\$[\*\*]) per claim. AMT will annually present evidence, in the form of a statement in the annual diligence report to ST. JUDE that such coverage is being maintained. Upon ST. JUDE'S request, AMT will furnish ST. JUDE with a Certificate of Insurance of each insurance policy obtained. ST. JUDE and ALSAC shall be listed as additional insured's in AMT's said insurance policies. If such insurance is underwritten on a 'claims made' basis, AMT agrees that any change in underwriters during the term of this Agreement and thereafter so long as Licensed Products are being sold will require the purchase of 'prior acts' coverage to ensure that coverage will be continuous throughout the term of this Agreement and thereafter so long as Licensed Products are being sold.

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9. **TERM AND TERMINATION**

9.1 Term. This Agreement shall commence on the Effective Date and, and unless sooner terminated in accordance with any of the provisions herein, expire when no further payment is due from AMT to ST. JUDE hereunder in relation to sales of Licensed Product.

9.2 Voluntary Termination by AMT. AMT shall have the right to terminate this Agreement, for any reason, upon ninety (90) days, prior written notice to ST. JUDE. Upon termination, a final report as described in Section 5.2 shall be submitted and any previously arising (before the effective termination date) milestone payments, annual fees, royalty payments, and unreimbursed patent expenses due to ST. JUDE shall become immediately payable.

9.3 **Termination for Default.** In the event that either Party commits a material breach of its obligations under this Agreement and fails to cure that breach within [\*\*] days after receiving written notice thereof, the other Party may terminate this Agreement immediately upon written notice to the party in breach unless the Party allegedly in breach disputes that a material breach has occurred and submits notice of such dispute to the other Party, following which the Parties shall first try to resolve the dispute within [\*\*] days of such notice and if such dispute cannot be resolved, the dispute shall be subject to the jurisdiction of the courts pursuant to Section 10.7. For the avoidance of doubt, there shall be no termination of this Agreement pending the outcome of dispute resolution.

If the alleged breach involves non payment of any undisputed amounts due ST. JUDE under this Agreement, AMT shall pay an interest penalty based on the amount owed at a daily accrual rate equal to the lesser of [\*\*] percent ([\*\*]%) per annum or the highest rate permissible by law on the unpaid balance until the undisputed amount is paid in full.

9.4 **Termination for Insolvency.** ST. JUDE shall have the right to terminate this Agreement on written notice to AMT in the event of the occurrence of insolvency of AMT.

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9.5 **Effect of Termination on Sublicensees.** If termination under Sections 9.3 or 9.4 of this Agreement is no fault of a Sublicensee ST. JUDE agrees to enter into a direct license of Patent Rights with any Sublicensee on substantially the same terms as the sublicense between AMT and the Sublicensee with respect to terms pertaining to the Patent Rights, provided that the terms of the sublicense are at least as favorable to ST. JUDE as the terms of this Agreement and prior to the making of any such sub-license by AMT, ST. JUDE will undertake directly to such Sublicensee that ST. JUDE will do this.

9.6 **Effect of Termination.** Upon termination, AMT shall cease to utilize Patent Rights and shall so certify to ST. JUDE in writing that Patent Rights are not being used for any purpose by AMT. Termination shall not affect any rights or obligations which have accrued prior to termination or which by their nature are intended to survive termination such as Section 1, 5.2 (obligation to provide final report and payment), 7, 8.1, 9.5, 10.1, 10.7 and 10.8. Upon the early termination of this Agreement, AMT may complete and sell any work-in-progress and inventory of Licensed Products that exists as of the effective date of termination, provided that (i) AMT is current in payment of all amounts due ST. JUDE under this Agreement, (ii) AMT pays ST. JUDE the applicable royalty on such sales of Licensed Products in accordance with the terms and conditions of this Agreement, and (iii) AMT shall complete and sell all work-in-progress and inventory of Licensed Products within [\*\*] months after the effective date of termination.

## 10. MISCELLANEOUS

10.1 Representation and Warranties of both Parties.

- (i) Each Party hereby represents and warrants to the other Party, as of the Effective Date, as follows:
  - (a) Such Party has the power and authority and legal right to enter into this Agreement, to perform its obligations and to grant the licenses hereunder,
- (ii) Further Representations and Warranties of ST. JUDE:
  - (a) ST. JUDE either legally or beneficially owns or controls the entire right, title and interest in and to the Patent Rights licensed hereunder.
  - (b) there is, to its knowledge as of the Effective Date, no action, suit, claim, proceeding or governmental investigation pending or threatened against ST. JUDE with respect to enforceability of the Patent Rights licensed hereunder, either at law or in equity, before any court or administrative

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and has taken all necessary action on its part to authorize the execution and delivery of this Agreement and the performance of its obligations hereunder;

- (b) This Agreement has been duly executed and delivered on behalf of such Party and constitutes a legal and valid obligation binding upon such Party and enforceable against it in accordance with its terms;
- (c) The execution, delivery and performance of this Agreement by such Party do not conflict with any agreement, instrument or understanding, oral or written, to which it is a party or by which it is bound, nor violate any applicable law or regulation of any governmental body or administrative or other agency having jurisdiction over it;
- (d) Such Party is aware of no action, suit, inquiry or investigation instituted by any third party that questions or threatens the validity of this Agreement; and
- (e) All necessary consents, approvals and authorizations of all governmental authorities and other persons required to be obtained by such Party in connection with this Agreement have been obtained.

agency or before any governmental department, commission, board, bureau, agency or instrumentality, whether United States or foreign.

- (c) ST. JUDE has informed AMT in writing of all intellectual property rights of third parties in the Field of which ST. JUDE is aware to the best of ST. JUDE's knowledge.

ST. JUDE MAKES NO OTHER WARRANTIES CONCERNING THE PATENT RIGHTS, INCLUDING WITHOUT LIMITATION ANY EXPRESS OR IMPLIED WARRANTY OF MERCHANTABILITY OR FITNESS FOR A PARTICULAR PURPOSE. Specifically, ST. JUDE makes no warranty or representation (i) regarding the validity or scope of the Patent Rights, (ii) that exploitation of the Patent Rights or any Licensed Product will not infringe any patents or other intellectual property rights of a third party, and (iii) that any third party is not currently infringing or will not infringe the Patent Rights.

- 10.2 Force Majeure. Neither Party will be responsible for its inability to perform any of its obligations under this Agreement resulting from causes beyond the reasonable control of such Party, including without limitation fires, explosion, flood, war, strike, or riot, provided that the nonperforming Party uses reasonable efforts to avoid or remove such causes of nonperformance and continues performance under this Agreement with reasonable dispatch whenever such causes are removed.
- 10.3 Headings. All headings are for convenience only and shall not affect the meaning of any provision of this Agreement.
- 10.4 Binding Effect. This Agreement shall be binding upon and inure to the benefit of the parties and their respective permitted successors and assigns.

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- 10.5 Assignment. The benefit and/or burden of this Agreement may not be assigned by either Party without the prior written consent of the other Party, such consent not to be unreasonably withheld, except that AMT may, without the consent of ST. JUDE assign this Agreement to an Affiliate or to a successor in connection with a merger, consolidation, or sale of all or substantially all of its assets or that portion of its business to which this Agreement relates, but shall notify ST. JUDE of such an assignment within [\*\*] days of its occurrence. Any assignment in violation of this provision shall be null and void.
- 10.6 Amendment and Waiver. This Agreement may be amended, supplemented, or otherwise modified only by means of a written instrument signed by both Parties. Any waiver of any rights or failure to act in a specific instance shall relate only to such instance and shall not be construed as an agreement to waive any rights or fail to act in any other instance, whether or not similar.
- 10.7 Governing Law. This Agreement shall be governed by and construed in accordance with the laws of the State of New York irrespective of any conflicts of law principles or choice of law rules of any state or country. Any lawsuit that may be brought with respect to this Agreement shall be brought and tried in a court of competent jurisdiction in New York. AMT represents that choice of law provisions agreed to by parties to a written contract are generally honored under Dutch law.
- 10.8 Notice. Any notices required or permitted under this Agreement shall be in writing, shall specifically refer to this Agreement, and shall be sent by hand, recognized national overnight courier, confirmed facsimile transmission, or registered or certified mail, postage prepaid, return receipt requested, to the following address or facsimile numbers of the parties:

To ST. JUDE:                      Office of Technology Licensing  
   Mail Stop 0742  
   St. Jude Children's Research Hospital

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332 North Lauderdale  
Memphis, Tennessee 38105 Attn: Director

Facsimile: (901) 495-3148

To AMT:                              Amsterdam Molecular Therapeutics B.V.  
   Meibergdreef 611105 BA  
   Amsterdam, The Netherlands  
   Attn: Anthony Gringeri

Facsimile: +31 20 566 9272

All notices under this Agreement shall be deemed effective upon receipt. A Party may change its contact information upon written notice to the other Party.

- 10.9 Severability. In the event that any provision of this Agreement shall be held invalid or unenforceable for any reason, such invalidity or unenforceability shall not affect any other provision of this Agreement, and the Parties shall negotiate in good faith to modify the Agreement to preserve (to the extent possible) their original intent. During such negotiation, this Agreement shall be construed as if such provision were deleted by agreement of the Parties.
- 10.10 Entire Agreement. This Agreement, together with the Sponsored Research Agreement and the AMT Technology License Agreement between the Parties executed concurrently herewith, constitutes the entire agreement between the Parties with respect to the subject matter and supersedes all prior agreements or understandings between the Parties relating to its subject matter.

IN WITNESS WHEREOF, the Parties have caused this Agreement to be executed by their duly authorized representatives.

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Amsterdam Molecular Therapeutics B.V.

St. Jude Children's Research Hospital, Inc.

By: /s/ A.J. Gringeri

By: /s/ J. Scott Elmer

Date: 4 July 2008

Date: 07/07/08

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ST. JUDE CHILDREN’S RESEARCH HOSPITAL, INC. (1)

and

UNIQUE BIOPHARMA BV (2)

AMENDMENT N°1 TO THE  
EXCLUSIVE LICENSE AGREEMENT

**THIS AMENDMENT N°1 TO THE EXCLUSIVE LICENSE AGREEMENT** (this “Amendment”), with the effective date of July 12, 2012 (“Effective Date”),

**BY AND BETWEEN**

- (1) **ST. JUDE CHILDREN’S RESEARCH HOSPITAL, INC.**, a Tennessee not-for-profit corporation located at 262 Danny Thomas Place, Memphis, Tennessee 38105 (“St. Jude”); and
- (2) **UNIQUE BIOPHARMA BV** (formerly: Amsterdam Molecular Therapeutics (AMT) B.V.), a company incorporated under the laws of the Netherlands, with offices at Meibergdreef 61, 1105 BA Amsterdam, The Netherlands (“uniQure”).

(each, a “Party” and together the “Parties”)

**BACKGROUND:**

- (A) The Parties have signed an Exclusive License Agreement dated July 7th, 2008 (hereinafter the “Agreement”).
- (B) The Parties desire that the Agreement be amended as set forth below in order to:
- I. change the name of the licensee from Amsterdam Molecular Therapeutics B.V. (“AMT”) to uniQure biopharma B.V. This name change is the result of a transaction that took place on 30 March 2012, whereby Amsterdam Molecular Therapeutics N.V., a public company, was liquidated and all its operations and stocks were transferred to UniQure B.V., a privately held company;
  - II. add language related to financial terms associated with sublicensing, so as to clarify financial obligations due to St.Jude from sublicensing of the patent rights granted in the Agreement by uniQure in order to expedite the development of therapeutics for rare diseases.

**IT IS NOW AGREED AS FOLLOWS:**

**1. Modifications**

- I. In the Agreement, all references to “Amsterdam Molecular Therapeutics B.V.” are changed to “uniQure biopharma B.V.”.
- II. In the Agreement, all references to “AMT” are changed to “uniQure”.
- III. Section 4.5 (i)\* is amended to read as follows:

\* 2<sup>nd</sup> subsection (f) of Section 4.5.

- (i) Support for research, Development and/or manufacturing activities corresponding directly to the Development and commercial manufacture of Licensed Products, which do not exceed the fully-burdened cost for undertaking such research, Development, and/or manufacturing performed by or for AMT (including third parties on AMT’s behalf), each pursuant to a specific agreement including a performance plan and commensurate budget;
- IV. The following Section 4.7 is added to the Agreement:

4.7 Sublicense consideration apportionment. The percentages referred to under subsections (i), (ii) and (iii) immediately below the first paragraph of Section 4.5 shall apply only to that portion of sublicense consideration attributable to sublicensing of the Patent Rights. In any agreement which includes the grant of a sublicense to Patent Rights along with other rights and assets held by uniQure that are necessary or desirable for the development, manufacture and sale of Licensed Products, the Parties shall agree on the portion of income from such an agreement that should be attributable to sublicensing of the Patent Rights, taking into account the value of the Patent Rights in comparison to the value of the other rights and assets transferred by uniQure to the sublicensee that are necessary or desirable for the development, manufacture and sale of Licensed Products.

2. **Miscellaneous:** All the other provisions of the Agreement remain unchanged and fully applicable between the Parties, and the terms and definitions used in the Agreement shall, so far as possible, apply to this Amendment.

**IN WITNESS WHEREOF**, the Parties have caused this Agreement to be executed as of the Effective Date.

St. Jude Children's Research Hospital, Inc.

UniQure biopharma B.V.

By: \_\_\_\_\_  
Name: \_\_\_\_\_  
Title: \_\_\_\_\_  
Date: \_\_\_\_\_

By: /s/ PJ Morgan  
Name: PJ Morgan  
Title: CFO  
Date: 12 July 2012

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Confidential Materials omitted and filed separately with the Securities and Exchange Commission. Double asterisks denote omissions.

## CO-DEVELOPMENT AND LICENSE AGREEMENT

This Co-Development and License Agreement (this “Agreement”) is entered into as of 29 April 2013 (the “Effective Date”), by and between uniQure Biopharma B.V., formerly known as Amsterdam Molecular Therapeutics (AMT) B.V., a Dutch corporation, with its offices at Meibergdreef 61, 1105 BA Amsterdam, The Netherlands (“uniQure”), and Chiesi Farmaceutici S.p.A., an Italian corporation, with its offices at Via Palermo, 26/A, 43122 Parma, Italy (“Chiesi”). uniQure and Chiesi are sometimes referred to herein individually as a “Party” and collectively as the “Parties”.

WHEREAS, uniQure is a company engaged in the research and clinical development of human gene based therapies and uniQure Controls (as defined below) all relevant rights to a certain Gene Therapy Product for Hemophilia B, as more specifically described below;

WHEREAS, Chiesi is a pharmaceutical company engaged in the research, development, manufacture and commercialization of products for human diseases and disorders;

WHEREAS, Chiesi and uniQure are interested in collaborating in the co-development and future Commercialization (as defined below) of the Product, on the terms and conditions set forth herein.

NOW, THEREFORE, uniQure and Chiesi hereby agree as follows:

### ARTICLE I DEFINITIONS; INTERPRETATION

Capitalized terms used herein shall have the meanings assigned to them as follows.

Section 1.1 “AAV5 Vector” shall mean a recombinant adeno-associated virus vector with the serotype 5 that is a non-pathogenic, replication defective, parvovirus engineered to deliver functional gene copies to humans.

Section 1.2 “Acquired Party” has the meaning set forth in Section 15.1.

Section 1.3 “Acquirer” has the meaning set forth in Section 15.1.

Section 1.4 “Additional Rights” has the meaning set forth in Section 7.6(a).

Section 1.5 “Additional Rights Agreement” shall mean a written agreement to which either or both Parties are a party and that conveys rights in Additional Rights that are included in Licensed Technology or the Chiesi Technology pursuant to Section 7.6(d).

Section 1.6 “Affiliate” shall mean, with respect to a Party, any Person Controlled by, in Control of, or under common Control with such Party.

Section 1.7 “Agreement” has the meaning set forth in the first and opening paragraph of this Agreement.

Section 1.8 “Alliance Manager” has the meaning set forth in Section 2.2(f).

Section 1.9 “Applicable Laws” shall mean all applicable laws, rules and regulations, including any rules, regulations, guidelines or other requirements of any Regulatory Authority, that may be in effect from time to time.

Section 1.10 “Approved Activities” shall mean those activities identified on Exhibit C.

Section 1.11 “Arbitration Request” has the meaning set forth in Section 13.2(b)(i).

Section 1.12 “Average Net Sales Price” shall mean the average net sales price of a particular Product in the Territory, calculated on a monthly basis, by dividing the Net Sales of the Product in the Territory effected in a particular calendar month by the number of patient doses of the Product accounting for the Net Sales in such calendar month.

Section 1.13 “Breaching Party” has the meaning set forth in Section 12.3.

Section 1.14 “Business Day” shall mean a day on which banking institutions in Amsterdam, The Netherlands and Parma, Italy, are open for business, excluding any Saturday or Sunday.

Section 1.15 “Change of Control” shall mean, with respect to a Party, the acquisition by any Third Party, directly or indirectly, of the Control of such Party.

Section 1.16 “Chiesi” has the meaning set forth in the first and opening paragraph of this Agreement.

Section 1.17 “Chiesi Know-How” shall mean all Know-How Controlled by Chiesi as of the Effective Date or during the Term that is necessary or useful for the Development, use, Manufacture, having Manufactured, or Commercialization of the Product. Chiesi Know-How also includes any Joint Know-How to the extent Controlled by Chiesi, and such other Know-How included in the Chiesi Know-How pursuant to Section 7.6.

Section 1.18 “Chiesi Patents” shall mean all Patent Rights Controlled by Chiesi as of the Effective Date or during the Term that are directed to any Chiesi Know-How or the Development, Manufacture, having Manufactured, use, or Commercialization of the Product. Chiesi Patents also include any Joint Patents to the extent Controlled by Chiesi, and such other Patent Rights included in the Chiesi Patents pursuant to Section 7.6.

Section 1.19 “Chiesi Shared Costs” has the meaning set forth in Section 8.2(a).

Section 1.20 “Chiesi Sole Costs” shall mean all costs incurred in connection with launch and pre-launch commercial activities for the Product in the Territory, including key opinion leader (KOL) development, market research, pricing and reimbursement studies.

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Section 1.21 “Chiesi Technology” shall mean Chiesi Know-How and Chiesi Patents.

Section 1.22 “Claims” has the meaning set forth in Section 14.1.

Section 1.23 “CMC” shall mean the Chemistry, Manufacturing and Controls portion of any IMPD or Marketing Authorization Application.

Section 1.24 “Collaboration” shall mean the relationship between and activities conducted by the Parties under this Agreement and all other agreements between the Parties referenced herein (other than the Confidentiality Agreement), including the Commercialization Agreement (collectively, the “Collaboration Agreements”).

Section 1.25 “Collaboration Agreements” has the meaning set forth in Section 1.24.

Section 1.26 “Commercialization Agreement” shall mean that certain Commercialization Agreement for Glybera concluded separately between the Parties on the date hereof.

Section 1.27 “Commercialization” shall mean any and all activities, whether before or after Regulatory Approval, directed to the marketing, detailing and promotion of the Product and shall include pre-launch, launch and post-launch marketing, promoting, detailing, marketing research, medical affairs, managed markets, distributing, offering to commercially sell and commercially selling the Product, importing, exporting or transporting the Product for commercial sale and regulatory affairs with respect to the foregoing, including the filing and obtaining of Price and Reimbursement Approval for the Product, but shall not include Manufacturing nor any Development activities. When used as a verb, “Commercializing”, “Commercialize” and “Commercialized” shall mean to engage in Commercialization.

Section 1.28 “Commercially Reasonable Efforts” shall mean, with respect to the efforts to be expended by a Party with respect to a goal, reasonable, diligent, good faith efforts to accomplish such goal as a similarly situated (with respect to size, stage of development, and assets) biotechnology or pharmaceutical company, as the case may be, would use to accomplish a similar goal under similar circumstances so as to achieve such goal as expeditiously as possible; provided that, with respect to the Development and Commercialization of the Product, such efforts shall be substantially equivalent to those efforts and resources that a similarly situated (with respect to size, stage of development, and assets) biotechnology or pharmaceutical company, as the case may be, would typically devote to its own internally discovered products of similar market potential at a similar stage in their development or product life so as to achieve such goal as expeditiously as possible (which, with respect to activities for which Chiesi is responsible, shall be without regard to any amounts paid or payable to uniQure with respect to the Product under this Agreement, the Commercialization Agreement, or the HemB Supply and Distribution Agreement).

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Section 1.29 “Competing Product” shall mean any Gene Therapy product developed, manufactured or commercialized in the Field other than the Product Developed, Manufactured or Commercialized pursuant to this Agreement.

Section 1.30 “Competitive Infringement” has the meaning set forth in Section 9.3(a).

Section 1.31 “Confidential Information” shall mean all confidential or proprietary information of a Party, including information regarding such Party’s or its Affiliates’ or licensors’ technology, products, business, business plans, financial status, biological substances, chemical substances, formulations, techniques, methodology, equipment, sources of supply and patent positioning and information belonging to such Party’s Affiliate or a Third Party provided to the other Party under this Agreement. The status, prospects or objectives regarding the Development Program or any Product being developed hereunder, as well as the terms and conditions of this Agreement, shall be deemed “Confidential Information” of both Parties. All information disclosed by uniQure prior to the Effective Date pursuant to the Two Way Confidentiality Disclosure Agreement between Amsterdam Molecular Therapeutics (AMT) B.V. and Chiesi Farmaceutici S.p.A., dated 22 July 2010 (the “Confidentiality Agreement”) shall be deemed “Confidential Information” of uniQure hereunder.

Section 1.32 “Confidentiality Agreement” has the meaning set forth in Section 1.31.

Section 1.33 “Control” or “Controlled” shall mean (a) when used in reference to any Confidential Information, Know-How, Patent or other intellectual property rights, the possession (whether by ownership or license (other than solely pursuant to a license under this Agreement)) by such Party or any of its Affiliates, of the legal authority or right to grant to the other Party access or a license or sublicense to such Confidential Information, Know-How, Patent or other intellectual property rights as provided herein, without violating the terms of any agreement or arrangement with any Third Party, or (b) when used in reference to Section 1.6, Section 1.15 and Section 15.1, (i) the possession, directly or indirectly, of the power to direct the management or policies of a Person, whether through ownership of voting securities, by contract or otherwise; (ii) ownership of fifty percent (50%) or more of the voting securities entitled to vote for the election of directors in the case of a corporation, or fifty percent (50%) or more of the equity interest in the case of any other type of legal entity; or (iii) status as a general partner in any partnership, or any other arrangement whereby a Person controls or have the right to control the board of directors or equivalent governing body of a corporation or other Person. Notwithstanding the foregoing, any portfolio company of any stockholder of such Person (which stockholder is a venture capital fund or private equity fund) shall not be deemed to be “under common Control with” such Person.

Section 1.34 “Controlling Party” has the meaning set forth in Section 7.6(b).

Section 1.35 “Cover” or “Covered” shall mean, with respect to any Patent and the subject matter at issue, that, but for a license granted under a Valid Claim of such Patent, the manufacture, use, sale, offer for sale or importation of the subject matter at issue would infringe such Valid Claim, or, in the case of a

patent application, would infringe a Valid Claim in such patent application if it were to issue as a patent.

Section 1.36 “Development” shall mean all non-clinical and clinical drug development activities, each to the extent reasonably relating to the development of the Product in the Territory. Development shall include toxicology, pharmacology, and other non-clinical efforts, test method development and stability testing, formulation development, delivery system development, quality assurance and quality control development, statistical analysis, the conduct of clinical trials or other activities, including regulatory activities, relating to obtaining Regulatory Approval. When used as a verb, “Develop” means to engage in Development activities.

Section 1.37 “Development Plan and Budget” shall mean a plan and budget setting forth the Development activities with respect to the Product and the budget therefor, as prepared, updated and amended from time to time in accordance with this Agreement. The Development Plan and Budget includes the Initial Development Plan and Budget.

Section 1.38 “Development Program” shall mean the pre-clinical, CMC, clinical and other development, regulatory and other pre-Marketing Authorization commercial activities of the Parties directed to the Product and undertaken in accordance with the Development Plan and Budget.

Section 1.39 “Effective Date” has the meaning set forth in the first and opening paragraph of this Agreement.

Section 1.40 “EMA” shall mean the European Medicines Agency and any successor agency thereto.

Section 1.41 “EPO” has the meaning set forth in Section 9.2(d)(iii).

Section 1.42 “EU” shall mean the European Union.

Section 1.43 “EU Member States” shall mean Austria, Belgium, Bulgaria, Cyprus, the Czech Republic, Denmark, Estonia, Finland, France, Germany, Greece, Hungary, Ireland, Italy, Latvia, Lithuania, Luxembourg, Malta, the Netherlands, Poland, Portugal, Romania, Slovakia, Slovenia, Spain, Sweden and the United Kingdom.

Section 1.44 “Executive Officers” shall mean the Chief Executive Officer of Chiesi or a senior officer designated by Chiesi, and the Chief Executive Officer of uniQure or a senior officer designated by uniQure.

Section 1.45 “Existing Third Party Licenses” shall mean the PHS Agreements, the PSC Agreement and the St. Jude Agreements.

Section 1.46 “FDA” shall mean the US Food and Drug Administration and any successor agency thereto.

Section 1.47 “Field” shall mean the treatment of Hemophilia B in humans.

Section 1.48 “First Commercial Sale” shall mean, the first sale by Chiesi, an Affiliate of Chiesi, or a Sub-distributor of Chiesi, as the case may be, of the Product to a Third Party in the Territory; provided, however, that neither (a) transfers of the Product between Chiesi and its Affiliates, or Sub-distributors nor (b) supply of the Product for clinical trial purposes, shall constitute a First Commercial Sale.

Section 1.49 “FTE” shall mean a full time equivalent person year (consisting of a total of [\*\*] hours per year) of work on or directly related to activities undertaken by uniQure hereunder and related management activities.

Section 1.50 “FTE Rate” shall mean EUR [\*\*] per FTE, which includes overhead expenses and bench fees (materials used during Development, but excluding expenses for materials and external costs for GMP manufacturing).

Section 1.51 “Fully Loaded Cost of Goods” shall mean the fully loaded cost of goods of the Product as defined in Exhibit D.

Section 1.52 “Gene Therapy” shall mean the introduction and expression of genetic material in cells of a person in order to cure a disease or to minimize disease symptoms.

Section 1.53 “HemB Supply and Distribution Agreement” has the meaning set forth in Section 3.4.

Section 1.54 “HHS” has the meaning set forth in Section 1.91.

Section 1.55 “ICC” shall mean the International Chamber of Commerce.

Section 1.56 “IMPD” shall mean an application submitted to a Regulatory Authority to initiate human clinical trials, including (a) an Investigational Medicinal Product Dossier required to be submitted to the EMA or other Regulatory Authorities in the EU Member States for Regulatory Approval of clinical trials in the EU Member States, as further defined in the Clinical Trials Directive (2001/20/EC), (b) any non-EU Member States equivalent of the foregoing in any other country, and (c) all supplements and amendments that may be filed with respect to the foregoing.

Section 1.57 “Improvement” shall mean any invention, discovery, development or modification relating to the Licensed Technology or the Product or the development, manufacture or exploitation thereof, including any method or process of manufacturing or using the Product, and any formulation for the Product, whether or not patentable as well as packaging and labeling of the Product, in each case if and to the extent Controlled by uniQure during the Term.

Section 1.58 “In-Person JDC Meetings” has the meaning set forth in Section 2.2(c)(i).



Section 1.59 “Indemnified Party” has the meaning set forth in Section 14.3(a).

Section 1.60 “Indemnifying Party” has the meaning set forth in Section 14.3(a).

Section 1.61 “Initial Development Plan and Budget” shall mean the activities and budget set forth on Exhibit A attached hereto.

Section 1.62 “Invalidity Claim” has the meaning set forth in Section 9.5.

Section 1.63 “JCC” has the meaning set forth in Section 2.3(a).

Section 1.64 “JDC” has the meaning set forth in Section 2.2(a).

Section 1.65 “Joint Know-How” has the meaning set forth in Section 9.1(c).

Section 1.66 “Joint Patents” shall mean all Patent Rights that are directed to any Joint Know-How.

Section 1.67 “JSC” has the meaning set forth in Section 2.1(a).

Section 1.68 “Know-How” shall mean all technical, scientific, manufacturing, financial, commercial and other know-how, data, tangible materials, information, trade secrets, ideas, formulae, inventions, discoveries, processes, machines, compositions of matter, formulations, improvements, protocols, techniques, works of authorship, and results of experimentation and testing (whether or not patentable) in written, electronic, physical (including in the form of tangible compounds or cell lines), oral or any other form.

Section 1.69 “License” has the meaning set forth in Section 7.1.

Section 1.70 “Licensed Know-How” shall mean all Know-How Controlled by uniQure as of the Effective Date or during the Term that is necessary or useful to Develop, use, or Commercialize the Product in the Field in the Territory. Licensed Know-How also includes any Joint Know-How to the extent Controlled by uniQure, and such other Know-How included in the Licensed Know-How pursuant to Section 7.6(c).

Section 1.71 “Licensed Patents” shall mean (a) all Patent Rights Controlled by uniQure as of the Effective Date or during the Term that are necessary or useful to Develop, use, or Commercialize the Product in the Field in the Territory, including those Patent Rights set forth in Exhibit E, which exhibit shall be updated or confirmed upon the date this Agreement has become effective pursuant to Section 12.1(b); (b) any Joint Patents to the extent Controlled by uniQure; and (c) such other Patent Rights included in the Licensed Patents pursuant to Section 7.6(c).

Section 1.72 “Licensed Technology” shall mean Licensed Know-How and Licensed Patents.

Section 1.73 “Losses” has the meaning set forth in Section 14.1.

Section 1.74 “Major Country” shall mean any of the following countries: France, Germany, Italy, Spain and the United Kingdom.

Section 1.75 “Manufacture” and “Manufacturing” shall mean all activities related to the production, manufacture, processing, filling, finishing, packaging, labeling, shipping and holding of the Product or any intermediate thereof, including

process development, process qualification and validation, scale up, pre-clinical, clinical and commercial manufacture and analytical development, product characterization, stability testing, quality assurance and quality control. When used as a verb, “Manufacture” shall mean to engage in Manufacturing activities.

Section 1.76 “Marketing Authorization” or “MA” shall mean the authorization issued by the relevant Regulatory Authority necessary to place on the market the Product in any country or regulatory jurisdiction in the Territory (including the approval of a Marketing Authorization Application in the EU Member States). For clarity, a Marketing Authorization shall not include any applicable Price and Reimbursement Approvals.

Section 1.77 “Marketing Authorization Application” or “MAA” shall mean an application submitted to a Regulatory Authority for marketing approval of a drug or biologic product, including (a) a Marketing Authorization Application in the EU Member States under Regulation (EC) No. 726/2004 or Directive 2001/83/EC, (b) any non-EU Member States equivalent of the foregoing in any other country in the Territory, and (c) all supplements and amendments that may be filed with respect to any of the foregoing.

Section 1.78 “Net Sales” shall mean the total amount of invoiced sales of the Product in the Territory by or on behalf of Chiesi or its Affiliates or Sub-distributors to Third Parties (including wholesalers, hospitals, end users and others), in bona fide arm’s length transactions, less the following deductions, in each case related specifically to the Product and customary in the trade and actually allowed and taken by such Third Parties and not otherwise recovered by or reimbursed to Chiesi: (a) cash discounts; (b) taxes on sales (such as sales or use taxes) to the extent added to the sale price and set forth separately as such in the total amount invoiced; (c) freight and insurance to the extent added to the sale price and set forth separately as such in the total amount invoiced; (d) amounts repaid or credited by reason of rejections, defects, recalls, expirations, or returns; and (e) any governmental mandated charge backs, rebates, and discounts. No deductions shall be made for (x) commissions paid to individuals, whether they are with independent sales agencies or regularly employed by Chiesi or any of its Affiliates, and on its payroll, (y) the cost of collections, and (z) any advertising and promotional expenses.

Section 1.79 “New Product” has the meaning set forth in Section 6.2(a).

Section 1.80 “NIH” has the meaning set forth in Section 1.92.

Section 1.81 “Non-Acquired Party” has the meaning set forth in Section 15.1.

Section 1.82 “Non-Arbitrable Termination Dispute” has the meaning set forth in Section 13.1(a).

Section 1.83 “Non-Breaching Party” has the meaning set forth in Section 12.3.

Section 1.84 “Non-Controlling Party” has the meaning set forth in Section 7.6(b).

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Section 1.85 “Party” and “Parties” has the meaning set forth in the first and opening paragraph of this Agreement.

Section 1.86 “Patent Challenge” has the meaning set forth in Section 12.4.

Section 1.87 “Patent Prosecution” shall mean the preparation, filing and prosecution of patent applications, and the maintenance of patents, included in Patent Rights.

Section 1.88 “Patent Right(s)” shall mean any patent or patent application, including utility patents, utility models, design patents, provisional applications, certificates of invention, and all divisionals, continuations, continuations-in-part, substitutions, reissues, reexaminations, renewals, extensions (including any supplemental patent certificate) or additions to any patent or patent application.

Section 1.89 “Paying Party” has the meaning set forth in Section 8.4.

Section 1.90 “Person” shall mean any natural person or any corporation, company, partnership, limited liability company, joint venture, firm, agency or other entity, including a Party.

Section 1.91 “Phase I/II Study” shall mean the first human clinical trial in which patients with Hemophilia B are dosed with the Product.

Section 1.92 “PHS” shall mean The National Institutes of Health (“NIH”) or the FDA, agencies of the US Public Health Service within the Department of Health and Human Services (“HHS”).

Section 1.93 “PHS Agreements” shall mean the PHS 2011 Agreement and the PHS 2007 Agreement.

Section 1.94 “PHS 2007 Agreement” shall mean the Non-Exclusive Patent License Agreement, number L-107-2007/0, dated as of 25 April/2 May 2007, by and between uniQure and PHS, as amended from time to time.

Section 1.95 “PHS 2011 Agreement” shall mean the Exclusive and Non-Exclusive Patent License Agreement, number L-116-2011/0, dated as of 5/10 August 2011, by and between uniQure and PHS, as amended from time to time.

Section 1.96 “Pivotal Study” shall mean, with respect to the Product, a human clinical trial, the principal purpose of which is to establish safety and efficacy of such Product in patients with Hemophilia B as required under Regulation (EC) No. 726/2004 or Directive 2001/83/EC, or a similar clinical trial prescribed by the applicable Regulatory Authority in the Territory. A Pivotal Study also includes any other human clinical trial intended as a pivotal study of such Product regarding Hemophilia B, such as a phase II/III or phase IIb clinical trial, whether or not such study is a traditional phase III clinical trial.

Section 1.97 “Pre-Existing Affiliate” has the meaning set forth in Section 15.1.

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Section 1.98 “Price and Reimbursement Approval” shall mean any approval or authorization of any Regulatory Authority establishing a pricing- and payment scheme or a reimbursement scheme for the Product in any country or jurisdiction of the Territory.

Section 1.99 “Product” shall mean a Gene Therapy product for the treatment of Hemophilia B in humans that includes an AAV5 Vector containing a functional copy of the codon-optimized human Factor IX gene or part thereof under the control of a liver-specific promoter.

Section 1.100 “Product Data” shall mean all preclinical and clinical data, safety data and all other supporting data, including pharmacology and biology data, regulatory documentation (including IMPDs, MAAs and other Marketing Authorizations, Regulatory Approvals and regulatory filings in the Territory) and other Know-How generated under the Development Program that relate to the Product.

Section 1.101 “Product Manufacturing Cost Reimbursement” has the meaning set forth on Exhibit D.

Section 1.102 “Product Transfer Price” has the meaning set forth on Exhibit D.

Section 1.103 “PSC” shall mean Protein Sciences Corporation.

Section 1.104 “PSC Agreement” shall mean the License Agreement, dated as of 22 March 2007, by and between uniQure and PSC, as amended from time to time.

Section 1.105 “Publishing Party” has the meaning set forth in Section 10.5(a).

Section 1.106 “Receiving Party” has the meaning set forth in Section 8.4.

Section 1.107 “Reconciliation Payment” has the meaning set forth in Section 8.2(d).

Section 1.108 “Regular JDC Meeting” has the meaning set forth in Section 2.2(c)(i).

Section 1.109 “Regulatory Approval” shall mean any and all approvals (including, where required, any applicable Price and Reimbursement Approvals), licenses, registrations or authorizations of any Regulatory Authority necessary for the Manufacture, use, and Commercialization of a Product in a country or jurisdiction, including IMPDs and Marketing Authorizations.

Section 1.110 “Regulatory Authority” shall mean any federal, national, multinational, state, provincial or local regulatory agency, department, bureau or other governmental entity with authority over the testing, Regulatory Approval, manufacture, use, storage, import, promotion, marketing or sale of a drug or biologic product in a country or jurisdiction, including the EMA.

Section 1.111 “SDEA” has the meaning set forth in Section 4.4.

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Section 1.112 “SEC” has the meaning set forth in Section 10.1(e).

Section 1.113 “Severed Clause” has the meaning set forth in Section 15.14.

Section 1.114 “Shared Costs” shall mean, for a calendar quarter, (a) each Party’s duly documented out-of-pocket costs incurred during such calendar quarter pursuant to the Development Program, including costs associated with preclinical studies, clinical studies, CRO, CMC, clinical supply and other reasonable and customary development expenses, as set forth in the Development Plan and Budget; (b) uniQure’s FTE Rate for each uniQure FTE conducting activities during such calendar quarter pursuant to the Development Program (including any activities conducted in connection with the Development Plan and Budget during the period from the Effective Date until the date this Agreement has become effective pursuant to Section 12.1(b)); (c) each Party’s out-of-pocket costs and expenses incurred during such calendar quarter associated with Patent Prosecution of Joint Patents in the Territory as provided in Section 9.2 and enforcement of Joint Patents against Competitive Infringement in the Territory as provided in Section 9.3, including (i) out-of-pocket costs incurred in gathering information or making filings with any governmental authority, (ii) fees and expenses of counsel and consultants (including translators) and (iii) extraordinary employee costs; (d) actual costs associated with any Approved Activity, whether incurred before or after the Effective Date, including as set forth in Exhibit C; (e) development and regulatory milestone payments associated with the Existing Third Party Licenses and, if required, any costs associated with any Additional Rights Agreements, to the extent incurred in connection with Development Program activities performed during such calendar quarter; (f) all costs and expenses relating to the withdrawal or recall of any Product in a country in the Territory prior to, Marketing Approval in such country pursuant to Section 4.3 and (g) all costs related to Phase IV (a post approval study) if agreed between the Parties. Shared Costs shall exclude uniQure Sole Costs and Chiesi Sole Costs.

Section 1.115 “St. Jude” shall mean St. Jude Children’s Research Hospital, Inc.

Section 1.116 “St. Jude Agreements” shall mean the Exclusive License Agreement and the Sponsored Research Agreement, both dated as of 7 July 2008, between St. Jude and uniQure, as amended from time to time.

Section 1.117 “Sub-distributor” shall mean a Third Party that is granted a sub-distribution or other Commercialization right in the Territory by Chiesi under this Agreement.

Section 1.118 “Subject Disclosure” has the meaning set forth in Section 10.3(b).

Section 1.119 “Supply Failure” has the meaning set forth in Section 3.5.

Section 1.120 “Term” has the meaning set forth in Section 12.1.

Section 1.121 “Territory” shall mean the EU Member States, Iceland, Liechtenstein and Norway as well as Albania, Algeria, Andorra, Bosnia, Brazil,

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Croatia, Egypt, Macedonia, Mexico, Monaco, Montenegro, Morocco, Pakistan, Republic of San Marino, Russia and ex-CIS countries (*i.e.* Armenia, Azerbaijan, Belarus, Georgia, Kazakhstan, Kirghizstan, Moldova, Tajikistan, Turkmenistan, Ukraine and Uzbekistan), Serbia (including Kosovo), Switzerland, Tunisia, Turkey, and Vatican City. For the avoidance of doubt, the Territory shall exclude China and the US.

Section 1.122 “Third Party” shall mean any Person other than uniQure, Chiesi, or their respective Affiliates.

Section 1.123 “uniQure” has the meaning set forth in the first and opening paragraph of this Agreement.

Section 1.124 “uniQure Shared Costs” has the meaning set forth in Section 8.2(a).

Section 1.125 “uniQure Sole Costs” shall mean (a) any expenses for extra patients in a clinical trial of the Product beyond the number required by Regulatory Authority guidelines, or through Regulatory Authority feedback, in the Territory, unless the inclusion of such extra patients is agreed to by the JDC, (b) post-Marketing Authorization Product Manufacturing costs unless specifically required by Chiesi, (c) any filing fees associated with CMC of the Product and (d) subject to Section 4.3, the costs of safety monitoring of the Product, including any filing fees associated with Pharmacovigilance.

Section 1.126 “Upfront Payment” has the meaning set forth in Section 8.1.

Section 1.127 “US” or “USA” shall mean the United States of America, including its territories and possessions.

Section 1.128 “Valid Claim” shall mean any claim within an issued and unexpired Patent or pending Patent application that (i) is not expired, lapsed, or abandoned, (ii) is not dedicated to the public, disclaimed, or admitted to be unenforceable or invalid; and (iii) has not been invalidated, held unenforceable or cancelled by a court or administrative agency of competent jurisdiction in an order or decision from which no appeal has been or can be taken, including through opposition, re-examination, reissue, disclaimer or otherwise.

Section 1.129 “WIPO” has the meaning set forth in Section 9.2(d)(iii).

Section 1.130 Interpretation. Any reference in this Agreement to an Article, Section, subsection, paragraph, clause, or Exhibit shall be deemed to be a reference to any Article, Section, subsection, paragraph, clause, or Exhibit, of or to, as the case may be, this Agreement. Except where the context clearly otherwise requires, (a) wherever used, the use of any gender will be applicable to all genders, (b) the singular shall include the plural and vice versa, (c) any definition of or reference to any agreement, instrument or other document refers to such agreement, instrument or other document as from time to time amended, supplemented or otherwise modified (subject to any restrictions on such amendments, supplements or modifications set forth herein or therein), (d) any reference to any Applicable Laws refers to such Applicable Laws as from time to time enacted, repealed or amended, (e) the words

“herein”, “hereof” and “hereunder”, and words of similar import, refer to this Agreement in its entirety and not to any particular provision hereof, (f) the words “include”, “includes” and “including” are deemed to be followed by the phrase “but not limited to”, “without limitation” or words of similar import, (g) the word “or” has the inclusive meaning (*i.e.*, “and/or”), (h) the word “day” means a calendar day, the word “month” means a calendar month, and the word “year” means, and the word “annual” refers to, a calendar year, (i) the word “quarterly” refers to a calendar quarter, (j) each accounting term used herein that is not specifically defined herein has the meaning given to it under the International Financial Reporting Standards, and (k) the captions or headings of the Exhibits, Articles, Sections or other subdivisions hereof are inserted only as a matter of convenience or for reference and shall have no effect on the meaning of the provisions hereof.

## ARTICLE II GOVERNANCE; DECISION MAKING

### Section 2.1 Joint Steering Committee.

(a) Formation and Membership. Within [\*\*] days after the Effective Date, Chiesi and uniQure shall establish a joint steering committee (the “JSC”) to manage the Collaboration. The JSC to be established under this Agreement shall be identical to the one to be established under the Commercialization Agreement. The JSC shall be comprised of [\*\*] executives or senior employees of Chiesi and [\*\*] executives or senior employees of uniQure with appropriate experience and level of decision-making authority. From time to time, in addition to the JDC described below, the Parties may establish one or more subcommittees of the JSC to oversee particular projects or activities (*e.g.*, activities under the Commercialization Agreement, financial reporting). Each such subcommittee shall be comprised of an equal number of representatives from each Party with appropriate experience and level of decision-making authority. Each subcommittee shall meet with a frequency to be agreed on by the Parties. Each Party may change any one or more of its representatives on the JSC or any subcommittee at any time upon written notice to the other Party.

(b) Responsibilities. The JSC shall be responsible for:

- (i) providing overall direction of the Collaboration;
- (ii) attempting to resolve disputes arising under the Collaboration Agreements; and
- (iii) performing such other tasks and undertaking such other responsibilities as may be set forth in the Collaboration Agreements.

(c) Meetings.

(i) The JSC shall meet at least [\*\*], by tele- or video-conference or in person, with the meetings in approximately [\*\*] to be held in-person. The location of in-person JSC meetings shall alternate between the headquarters offices of each Party, with the first meeting to take place at [\*\*].

(ii) Each Party shall use reasonable efforts to cause its representatives to attend the meetings of the JSC and any subcommittees. In addition, each Party may, at its discretion, invite a reasonable number of non-voting employees or officers, and, with the consent of the other Party, consultants or scientific advisors, to attend meetings of the JSC or any subcommittee, or the relevant portion thereof; provided that, its representatives and any such other employees, officers, consultants or scientific advisors are bound by written obligations of confidentiality that are at least as stringent as those set forth in this Agreement. Each Party shall bear all travel and living expenses of its representatives and other employees, officers, consultants or scientific advisors incurred to attend the meetings of the JSC or any subcommittee.

(iii) Either Party may also request, by providing written notice to the other Party, that a special meeting of the JSC be convened for the purpose of resolving disputes in connection with, or for the purpose of reviewing or making a decision pertaining to, any material matter within the purview of the JSC, the examination or resolution of which cannot reasonably be postponed until the next scheduled JSC meeting. Such meeting shall be convened at such time as may be mutually agreed upon by the Parties, but in any event shall be held within [\*\*] days after the date of such notice.

(d) Administrative Matters. The right to appoint the chairperson of the JSC shall alternate on an annual basis between Chiesi and uniQure, with [\*\*] having the right to appoint the chairperson for the first year of the Term. The Alliance Managers (defined below) shall work with the chairperson to develop JSC meeting agendas. The chairperson shall be responsible for calling meetings of the JSC and for leading the meetings. A JSC member of the chairing Party shall serve as secretary of such meetings. The secretary shall promptly prepare and distribute to all members of the JSC draft minutes of the meeting for review and comment, including a list of any actions or decisions approved by the JSC, with the goal of distributing final approved minutes of each JSC meeting within [\*\*] days after the meeting. Neither the chairperson nor the secretary shall have any greater authority than any other representative on the JSC and the Party appointing the chairperson and the secretary shall not have any greater authority than the other Party by virtue of its right to make such appointments. The chairperson shall include on the agenda any items proposed by either Party.

(e) Decision Making. Each Party, through its representatives, shall have one (1) vote on the JSC and each subcommittee. Both Parties must vote in the affirmative to allow the JSC or a subcommittee to take any action that requires the approval of the JSC or the subcommittee. Decision on any matter may be taken at a meeting, by teleconference, videoconference or by written agreement. If a subcommittee is unable to resolve any dispute, or to unanimously agree on any matter, within its responsibilities, such dispute or matter shall be referred to the JSC for resolution. Either Party may convene a special meeting of the JSC in accordance with Section 2.1(c)(iii) for the purpose of resolving any dispute within the JSC’s jurisdiction that represents a material issue the resolution of which cannot reasonably await until the next scheduled meeting of the JSC.

(f) Dispute Resolution by Executive Officers.

(i) If the JSC is unable to resolve any dispute within the responsibilities of the JSC specified in Section 2.1(b) within [\*\*] days after a Party provides notice to the other Party of the existence of such dispute, such dispute or other matter shall be referred to the Executive Officers for resolution, pursuant to Section 13.2.

(ii) In resolving any disputes under this Section 2.1(f), each Party shall act in good faith, subject to the terms and conditions of the Collaboration Agreements, and in a commercially reasonable manner without favoring other products being developed or commercialized by or on behalf of such Party or its Affiliates outside of the Collaboration.

Section 2.2 Joint Development Committee.

(a) Formation and Membership. Within [\*\*] days after the Effective Date, Chiesi and uniQure shall establish, as a subcommittee of the JSC, a joint development committee (the “JDC”) to manage the overall relationship between the Parties under this Agreement. The JDC shall be comprised of [\*\*] executives or senior employees of Chiesi and [\*\*] executives or senior employees of uniQure with appropriate experience and level of decision-making authority. From time to time, the Parties may establish one or more subcommittees of the JDC to oversee particular projects or activities (e.g., clinical/regulatory, CMC, development). Each such subcommittee shall be comprised of an equal number of representatives from each Party with appropriate experience and level of decision-making authority. Each subcommittee shall meet with a frequency to be agreed on by the Parties. Each Party may change any one or more of its representatives on the JDC or any subcommittee at any time upon written notice to the other Party.

(b) Responsibilities. The JDC shall be responsible for:

(i) periodically reviewing the Development Plan and Budget and suggesting or approving such updates or amendments to the Development Plan and Budget as the JDC deems appropriate, including all budgets relating to activities to be conducted hereunder and amendments thereto;

(ii) ensuring consistency and coordination between Development activities to be conducted by uniQure under the Development Plan and Budget and, if applicable, by Chiesi under the Development Plan and Budget;

(iii) providing overall strategic direction with respect to Development and regulatory activities for the Product, including activities conducted under the Development Plan and Budget;

(iv) overseeing Development and regulatory activities for the Product;

(v) discussing and addressing any supply chain or other delivery issues that have arisen or might arise relating to the Product;

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(vi) determining, managing and reviewing the Patent strategy relating to inventions made after the Effective Date covering the Product in the Territory;

(vii) attempting to resolve disputes arising under this Agreement that are referred to the JDC by either Party or any subcommittee; and

(viii) performing such other tasks and undertaking such other responsibilities as may be set forth in this Agreement or as may be delegated to it by the JSC.

(c) Meetings.

(i) The JDC shall meet at least [\*\*], by tele- or video-conference or in person, with the meetings in approximately [\*\*] to be held in-person (such [\*\*] regularly scheduled [\*\*] in-person meetings shall be the “Regular JDC Meetings”, while all in-person meetings of the JDC, including the Regular JDC Meetings, shall be “In-Person JDC Meetings”). The location of In-Person JDC Meetings shall alternate between the headquarters offices of each Party, with the first meeting to take place at [\*\*].

(ii) Each Party shall use reasonable efforts to cause its representatives to attend the meetings of the JDC and any subcommittees. In addition, each Party may, at its discretion, invite a reasonable number of non-voting employees or officers, and, with the consent of the other Party, consultants or scientific advisors, to attend meetings of the JDC or any subcommittee, or the relevant portion thereof; provided that, its representatives and any such other employees, officers, consultants or scientific advisors are bound by written obligations of confidentiality that are at least as stringent as those set forth in this Agreement. Each Party shall bear all travel and living expenses of its representatives and other employees, officers, consultants or scientific advisors incurred to attend the meetings of the JDC or any subcommittee.

(iii) Either Party may also request, by providing written notice to the other Party, that a special meeting of the JDC be convened for the purpose of resolving disputes in connection with, or for the purpose of reviewing or making a decision pertaining to, any material matter within the purview of the JDC, the examination or resolution of which cannot reasonably be postponed until the next scheduled JDC meeting. Such meeting shall be convened at such time as may be mutually agreed upon by the Parties, but in any event shall be held within [\*\*] days after the date of such notice.

(d) Administrative Matters. [\*\*] shall have the right to appoint the chairperson of the JDC. The Alliance Managers (defined below) shall work with the chairperson to develop JDC meeting agendas. The chairperson shall be responsible for calling meetings of the JDC and for leading the meetings. A [\*\*] JDC member shall serve as secretary of such meetings. The secretary shall promptly prepare and distribute to all members of the JDC draft minutes of the meeting for review and comment, including a list of any actions or decisions approved by the JDC, with the goal of distributing final approved minutes of each JDC meeting within [\*\*] days after the meeting. Neither the chairperson nor the secretary shall have any greater authority than any other representative on the JDC and [\*\*] shall not have any greater

authority than [\*\*] by virtue of its right to make such appointments. The chairperson shall include on the agenda any items proposed by either Party.

(e) **Decision Making.** Each Party, through its representatives, shall have one (1) vote on the JDC and each subcommittee. Both Parties must vote in the affirmative to allow the JDC or a subcommittee to take any action that requires the approval of the JDC or the subcommittee. Decision on any matter may be taken at a meeting, by teleconference, videoconference or by written agreement. If a subcommittee is unable to resolve any dispute, or to unanimously agree on any matter, within its responsibilities, such dispute or matter shall be referred to the JDC for resolution. Either Party may convene a special meeting of the JDC in accordance with Section 2.2(c)(iii) for the purpose of resolving any dispute within the JDC's jurisdiction that represents a material issue the resolution of which cannot reasonably await until the next scheduled meeting of the JDC.

(f) **Dispute Resolution.** If the JDC is unable to resolve any dispute within the responsibilities of the JDC specified in Section 2.2(b), or to unanimously agree on any matter set forth in subsections (i)-(vi) below, within [\*\*] days after a Party provides notice to the other Party of the existence of such dispute, then (A) uniQure shall have final decision-making authority (except as set forth below in this Section 2.2(f)) with respect to all research and Development activities with respect to the Product, with reasonable input from Chiesi taking into account Territory-specific matters, and (B) Chiesi shall have final decision-making authority with respect to all Commercialization activities with respect to the Product in the Territory, with reasonable input from uniQure taking into account uniQure's global Product strategy; provided, however, that the following decisions must be decided unanimously (or, if not able to be decided unanimously, will be referred to the JSC for resolution pursuant to Section 2.1), in that neither Party shall have the right to exercise its final decision-making authority to unilaterally:

- (i) increase the other Party's obligations or reduce such other Party's rights under this Agreement, including any obligation to conduct activities, or devote additional personnel to a specific activity to be conducted by such other Party, under the Development Plan and Budget, or require such other Party to conduct activities the costs of which are not reimbursed by such Party or included in Shared Costs;
- (ii) expand such Party's rights or reduce such Party's obligations under this Agreement;
- (iii) resolve disputes regarding the Parties' rights and obligations under this Agreement;
- (iv) make a decision that is expressly stated in this Agreement to require the other Party's prior approval or consent, or the mutual agreement of the Parties, or that is not consistent with the terms and conditions of this Agreement;
- (v) require the other Party to perform any act that such other Party reasonably believes to be inconsistent with Applicable Law; or

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(vi) make a decision in a manner that would cause a breach of any Existing Third Party License or Additional Rights Agreement, or to require any Third Party to take any actions not required to be performed by such Third Party under any Existing Third Party License or Additional Rights Agreement.

In addition, each Party shall exercise its final decision-making authority in good faith, subject to the terms and conditions of this Agreement, and in a commercially reasonable manner without favoring other products being developed or commercialized by or on behalf of such Party or its Affiliates outside the Collaboration. With respect to regulatory matters, both Parties agree that they will jointly work towards a regulatory strategy for the Product in the Territory, with an understanding that Chiesi (i) shall have the final decision right on the regulatory strategy for the Product in the Territory, including on all regulatory matters as described in Section 4.1 and Section 4.2 and (ii) shall support uniQure's global regulatory strategy for the Product unless such support leads to a material increase in costs or time to market for Chiesi, in which case Chiesi shall have the final decision right, as to cause the Parties to maintain the regulatory strategy for the Product in the Territory, as included in the Initial Development Plan and Budget as amended due to any mandatory request of any Regulatory Authority or Applicable Laws in the Territory. For the avoidance of doubt, "material increase in costs" shall mean additional costs (i.e. costs not reimbursed by uniQure to Chiesi) in excess of EUR [\*\*] and "material increase in time to market" shall mean an additional time for submission of a Marketing Authorization Application of more than [\*\*] days, in each case to be determined by comparing such additional costs or additional time with the initial costs or time included in the Initial Development Plan and Budget, as such initial costs or time may have been adjusted due to any mandatory request of any Regulatory Authority or Applicable Laws in the Territory (in other words, any increase of costs or time due to such mandatory requests shall not be relevant for the calculation of the EUR [\*\*] and [\*\*] days thresholds, whereas any other adjustments to the costs or time included in the Initial Development Plan and Budget shall be credited against such thresholds).

Without prejudice to the foregoing, it is further understood and agreed that the above final decision-making authority can be exercised by the respective representative(s) of uniQure or Chiesi, as the case may be, also at the JSC level.

Section 2.3 **Joint Commercialization Committee.** Within [\*\*] days after the Effective Date, Chiesi and uniQure may also establish, as a subcommittee of the JSC, a joint commercialization committee (the "JCC") to manage any specific matter not otherwise dealt with by the JDC hereunder. If a JCC is formed, the provisions of Sections 2.2(a) and 2.2(c) through (f) above shall apply to the JCC, *mutatis mutandis*.

Section 2.4 **Alliance Managers.** Each Party shall appoint an employee (or an employee of its Affiliate) to serve as an alliance manager ("Alliance Manager") with responsibility for overseeing that the Parties' activities are conducted in accordance with the Collaboration Agreements, and for being the primary point of contact between the Parties with respect to all such activities. The Alliance Managers to be appointed under this Agreement shall be identical to the ones to be appointed under the Commercialization Agreement. The Alliance Managers are responsible for driving the Collaboration progress and the resolution of issues between the Parties. The Alliance Managers may be members, but in any event may attend the meetings,

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of the JSC, JDC, or any other JSC subcommittee, and be responsible for communicating with and reporting to the JSC, JDC, and any other JSC subcommittee, on all relevant matters.

### ARTICLE III DEVELOPMENT; MANUFACTURE AND SUPPLY

(a) Subject to and in accordance with the terms and conditions of this Agreement, the Parties shall collaborate on the Development of the Product for the Territory in accordance with the Development Plan and Budget. Each Party shall be responsible for, and shall use Commercially Reasonable Efforts to conduct, those activities assigned to it under the Development Plan and Budget. Unless the Parties otherwise mutually agree:

(i) uniQure shall be responsible for, and shall use Commercially Reasonable Efforts to conduct all activities to Develop the Product in the Territory, including all clinical Development activities that are required to obtain Marketing Authorization in the Territory (with particular emphasis on each Major Country). The Parties will jointly work towards a regulatory strategy for the Product in the Territory, including preparing, filing, obtaining and maintaining all Regulatory Approvals necessary to Develop and Commercialize the Product in each Major Country, subject to Section 2.2(f) and, provided, that all matters under this sub-paragraph (i) shall be included in the Initial Development Plan and Budget; and

(ii) Subject to Section 2.2(f), Chiesi shall be responsible for, and shall use Commercially Reasonable Efforts to conduct all launch and pre-launch activities for the Product in the Territory, including KOL development, market research, and conducting pricing and reimbursement studies.

(b) Each successive Development Plan and Budget shall be prepared by uniQure in consultation with Chiesi, shall be reviewed and approved by the JDC, shall be consistent with the terms and conditions of the Agreement (including this Section 3.1) and shall specify among other things:

- (i) Development objectives,
- (ii) activities to be performed thereunder for at least the next [\*\*] years,
- (iii) associated budgets for the next year, and good faith projections for the [\*\*] years thereafter, regarding such activities,
- (iv) anticipated timelines for performance, and
- (v) specific deliverables.

(c) The Parties shall update the Development Plan and Budget [\*\*] and otherwise as reasonably required, as determined by the JDC. uniQure shall propose updates to the Development Plan and Budget in writing to Chiesi at least [\*\*] Business Days prior to each of the Regular JDC Meetings. Unless the Parties

otherwise agree, the number of FTEs of each Party for a given year of the Development Plan and Budget shall not exceed the applicable number of FTEs set forth in Exhibit F.

Section 3.2 Development Reports. Each Party shall provide written reports to the other Party at least [\*\*] Business Days before each Regular JDC Meeting, setting forth in reasonable detail such Party's and its Affiliates' activities and progress during the [\*\*] month period ending on the last day of [\*\*], as applicable, related to the Development of the Product.

Section 3.3 Development Program Costs. The costs of conducting the Development Program shall be borne by the Parties as set forth in Section 8.2.

Section 3.4 Manufacture of Product. uniQure shall provide supplies of the Product as necessary for the activities under the Development Plan and Budget; provided, however, that uniQure shall provide commercial supply of the Product pursuant to a supply and distribution agreement (the "HemB Supply and Distribution Agreement").

(a) Prior to the first dosing of the first patient in a Pivotal Study, the Parties shall negotiate in good faith the HemB Supply and Distribution Agreement, with the terms set forth on Exhibit D and other terms expected to be substantially similar to the Commercialization Agreement; provided, however, that the terms of the HemB Supply and Distribution Agreement, including the "Net Sales" definition and related provisions, may vary from those in the Commercialization Agreement in order to conform to and comply with the Existing Third Party Licenses and any applicable Additional Rights Agreements.

(b) uniQure may supply NIH with inert samples of the Product or its packaging for educational and display purposes.

Section 3.5 Failure to Supply.

(a) In the event that it becomes apparent to uniQure that it will be unable to provide supplies of the Product as necessary for the activities under the then current Development Plan and Budget ("Supply Failure"), uniQure shall, immediately after learning of such event or circumstances, notify Chiesi in writing of uniQure's Supply Failure, along with a reasonable explanation of the reason, to the extent then known to uniQure, for uniQure's Supply Failure and with a specific indication of the quantity of Product affected by such Supply Failure and anticipated timing of delivery of the Product. Promptly after Chiesi's receipt of any such notice, the Parties shall agree upon mutually acceptable revised quantities and delivery dates with respect to any ordered Product or, to the extent this is not possible in light of the specific or then unknown reason for uniQure's Supply Failure, shall discuss in good faith measures to further investigate the root cause and, as the case may be, appropriate steps to overcome such Supply Failure.

(b) Without prejudice to the foregoing paragraph (a), if

- (i) uniQure's Supply Failure affects at least supplies for a period of no less than nine (9) months, and

(ii) the reason for uniQure's Supply Failure could be established during the Parties' discussion pursuant to paragraph (a) above, and such reason was specifically related to uniQure's ability to Manufacture the Product at its current manufacturing site (i.e. the Supply Failure could reasonably be expected to be overcome if the Product was Manufactured at a different manufacturing site),

upon either Party's request, the Manufacturing of the Product shall be transferred to (A) uniQure's US manufacturing site, provided such site is operational at the relevant point in time, and further provided uniQure, within [\*\*] following such request, does not opt against such transfer, and (B) otherwise (i.e. if uniQure opts against such transfer within the foregoing [\*\*] period) to any other Third Party manufacturer mutually agreed to by uniQure and Chiesi. uniQure shall efficiently and promptly transfer to its US manufacturing site or, as the case may be, such Third Party manufacturer all information, licenses and rights controlled by uniQure and necessary to Manufacture the Product during the continuance of uniQure's Supply Failure. Such transfer shall ensure uniQure's ongoing control over the information, licenses and right so transferred, shall include the steps outlined in Exhibit G, and shall occur through email and videoconference interactions, as well as face-to-face meetings as required to ensure efficient transfer of technologies and capabilities.

If uniQure's US manufacturing site or, as the case may be, such Third Party manufacturer is unable to Manufacture the Product within [\*\*] months after uniQure has started the technology transfer to such person, Chiesi shall have the right to terminate this Agreement with three (3) month notice in writing, except if uniQure's Supply Failure is caused as a result of Force Majeure pursuant to Section 15.7. Such termination shall not become effective if, during such three (3) month notice period, uniQure has notified Chiesi of the ability of its US manufacturing site or, as the case may be, such Third Party manufacturer to Manufacture the Product. Upon termination of this Agreement by Chiesi pursuant to this Section 3.5(b), the provisions of Section 12.6 shall apply.

(c) Without prejudice to the foregoing paragraph (a), if

(i) uniQure's Supply Failure affects at least supplies for a period of no less than nine (9) months, and

(ii) the reason for uniQure's Supply Failure (A) could be established during the Parties' discussion pursuant to paragraph (a) above, but such reason was not specifically related to uniQure's ability to Manufacture the Product at its current manufacturing site (i.e. the Supply Failure could not reasonably be expected to be overcome if the Product was Manufactured at a different manufacturing site), or (B) could not be established during the Parties' discussion pursuant to paragraph (a) above during at least the foregoing nine (9) months period, and

(iii) uniQure's Supply Failure is not caused as a result of Force Majeure pursuant to Section 15.7,

Chiesi may terminate this Agreement with three (3) month notice in writing. Such termination shall not become effective if, during such three (3)

month notice period, uniQure has notified Chiesi of the end of its Supply Failure and has provided to Chiesi at least [\*\*] of the outstanding orders. Upon termination of this Agreement by Chiesi pursuant to this Section 3.5(c), the provisions of Section 12.6 shall apply.

#### ARTICLE IV REGULATORY MATTERS

Section 4.1 Regulatory Filings. Following the Effective Date, except as may be set forth in the HemB Supply and Distribution Agreement, (a) Chiesi shall own, and shall be responsible for preparing (with input from and in collaboration with uniQure pursuant to Section 3.1 and subject to Section 2.2(f)), filing and maintaining, all regulatory filings and Regulatory Approvals that are required for the Development, use, Manufacture or Commercialization of the Product in the Territory, and (b) Chiesi shall be responsible for all communications and interactions with Regulatory Authorities in the Territory with respect to the Development, use, Manufacture and Commercialization of the Product; provided that, at Chiesi's reasonable request, uniQure at its expense shall use Commercially Reasonable Efforts to provide assistance to Chiesi in the making of any such regulatory filings.

Section 4.2 Coordination. Subject to Section 2.2(f), the Parties shall coordinate their regulatory matters with respect to the Product in the Territory taking into account the framework of uniQure's global regulatory strategy for the Product. In particular:

(a) Each Party shall take such actions and otherwise cooperate with the other Party as may be reasonably requested by such other Party to enable such other Party to perform the activities assigned to it as set forth in the Development Plan and Budget, and any other Development or regulatory activities assigned to it under this Agreement, or to comply with any of such other Party's obligations under the Existing Third Party Licenses or any Additional Rights Agreement.

(b) Chiesi shall provide uniQure with electronic copies of all regulatory submissions to, and material communications with, Regulatory Authorities in the Territory and uniQure shall have the right to review and comment on such submissions and communications.

(c) Chiesi shall keep uniQure promptly (or to the extent possible, in advance) informed regarding Chiesi's (or its Affiliate's) regulatory strategy, planned regulatory submissions and material communications with Regulatory Authorities in the Territory with respect to the Product, including any changes to such strategy, submissions or communications.

(d) Chiesi shall provide uniQure with copies of any proposed regulatory submissions to, or material communications with, any Regulatory Authorities in the Territory with respect to the Product, at least [\*\*] days prior to submission.

(e) Chiesi shall promptly provide uniQure with copies of regulatory submissions to, and material communications with, any Regulatory Authorities in the Territory relating to the Product;

(f) uniQure shall have the right to have a senior employee of uniQure (expert for each relevant section of CTD) participate in all meetings or substantive teleconferences with the EMA and/or other Regulatory Authorities in the Territory with respect to the Product, as well as to participate in internal meetings or discussions of Chiesi occurring immediately before or after, and related to, such EMA and/or other Regulatory Authorities in the Territory meetings or teleconferences, and shall be provided with advance access to Chiesi's materials prepared for such EMA and/or other Regulatory Authorities in the Territory meetings and teleconferences.

(g) Chiesi shall provide uniQure, if feasible, with reasonable advance notice of any other meeting or substantive teleconference with Regulatory Authorities in the Territory relating to the Product.



(h) Without limiting the generality of any of the foregoing in this Section 4.2, Chiesi shall also promptly provide uniQure with an electronic copy of all material correspondence that Chiesi (or its Affiliate) receives from, or submits to, any Regulatory Authority in any country of the Territory related to the Product, including contact reports concerning conversations or substantive meetings, contact reports of all Regulatory Authority interactions in the Territory concerning conversations or substantive meetings, all required periodic reports, and cover letters of all agency submissions (including copies of all attachments to any such cover letters) relating to the Product. Chiesi shall also provide uniQure with any meeting minutes that Chiesi prepares that reflect communications with any Regulatory Authority in any country of the Territory regarding the Product.

Section 4.3 Product Withdrawals and Recalls. If any Regulatory Authority prior to Marketing Authorization in a country in the Territory (a) threatens, initiates or advises any action to remove the Product from the market in such country, or (b) requires or advises either Party or such Party's Affiliates to distribute a "Dear Doctor" letter or its equivalent regarding use of the Product in such country, then uniQure or Chiesi, as applicable, shall notify the other Party of such event within [\*\*] Business Days (or sooner if required by Applicable Law) after such Party becomes aware of the action, threat, advice or requirement. The JDC will meet promptly, but in any case within [\*\*] Business Days, to discuss and attempt to agree upon whether to recall or withdraw such Product in the Territory; provided, however, that if the Parties fail to agree within an appropriate time period or if the matter involves a safety issue that, in order to protect patient safety, does not allow for sufficient time for a discussion at the JDC level (in which event uniQure shall nonetheless provide advance notice and consultation with Chiesi to the maximum practical extent prior to making a decision), uniQure shall decide whether to recall or withdraw such Product in such country and shall undertake any such recall or withdrawal.

Section 4.4 Safety Monitoring; Pharmacovigilance. uniQure shall be responsible for safety monitoring of the Product and for any other obligations imposed by any Regulatory Authority in connection with the conduct of any preclinical or clinical activities or the granting of the relevant Marketing Authorization. The Parties shall negotiate and execute a detailed safety data exchange agreement (the "SDEA") prior to the start of clinical Development of the Product, to arrange any future pharmacovigilance exchange between the Parties when relevant. Each Party shall ensure, through its JDC representatives or designated personnel, that the competent pharmacovigilance or clinical groups or personnel from such Party

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begin to negotiate and establish the appropriate SDEA no later than [\*\*] months before the start of clinical Development of the Product, or on reasonable request of either Party. The SDEA shall be negotiated in good faith between the pharmacovigilance or clinical groups or personnel of each Party. The SDEA shall define the roles and responsibilities of each Party in terms of pharmacovigilance and define the detailed safety exchange required to permit compliance by each Party with safety reporting requirements to Regulatory Authorities and other entities in the Territory and ensure worldwide safety surveillance. At a minimum, uniQure may share pharmacovigilance information with its licensors and licensees of the Licensed Technology. In the event of a conflict between the terms of this Agreement and the SDEA, the terms of this Agreement shall govern.

## ARTICLE V COMMERCIALIZATION

Section 5.1 Overview. Subject to the terms and conditions hereunder, including Exhibit D, and the HemB Supply and Distribution Agreement, Chiesi will have sole right and responsibility to Commercialize the Product in the Field in the Territory, including for pre-launch Commercialization activities, including KOL development, pricing and reimbursement studies, as well as for post-launch Commercialization of the Product in the Field in the Territory, including all Chiesi Sole Costs relating thereto, and for booking all sales of the Product throughout the Territory.

## ARTICLE VI EXCLUSIVITY

Section 6.1 Exclusivity. During the Term, to the fullest extent consistent with any Applicable Law, neither Party nor, subject to Section 15.1, any of such Party's Affiliates, shall, by itself or through, with or on behalf of any Third Party, undertake the development, manufacture or commercialization anywhere in the Territory of any Competing Product.

## ARTICLE VII GRANT OF LICENSES

Section 7.1 uniQure License Grants. Subject to the Existing Third Party Licenses and the other terms and conditions of this Agreement, uniQure hereby grants to Chiesi and its Affiliates an exclusive right and license, with the right to grant sublicenses only to Sub-distributors, under the Licensed Technology, to co-Develop, use and Commercialize the Product in the Field in the Territory (the "License"). As used in the preceding sentence, "co-Develop" means that uniQure and its Affiliates may also exercise such Development rights in accordance with this Agreement, but that uniQure shall not grant any such rights to any Third Party in the Territory. Improvements that are (i) Controlled by uniQure or its Affiliates and (ii) necessary or useful to co-Develop, use or Commercialize the Product in the Field in the Territory, shall be deemed part of the Licensed Technology and included in the License. uniQure shall promptly disclose to Chiesi all Improvements that are Developed by uniQure or its Affiliates (alone or in collaboration with Chiesi or its Affiliates) during the Term and that are necessary or useful to co-Develop, use or Commercialize the Product in the Field in the Territory.

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Section 7.2 Chiesi License Grants.

(a) Subject to the terms and conditions of this Agreement, Chiesi hereby grants to uniQure and its Affiliates a non-exclusive, royalty-free, fully paid-up, irrevocable and perpetual (subject to Article XII) license in the Territory, with the right to grant sublicenses, under the Chiesi Technology, for the purposes of conducting Development Program activities.

(b) Subject to the terms and conditions of this Agreement, Chiesi hereby grants to uniQure a non-exclusive, worldwide, royalty-free, fully paid-up, irrevocable, perpetual (subject to Article XII) license, with the right to grant sublicenses, under the Chiesi Technology (including, subject to Section 7.3 any rights Chiesi may have in and to the Product Data), to Develop, Manufacture, having Manufactured, use, and Commercialize (outside the Territory) the Product in the Field.

Section 7.3 Use of Product Data Outside of Territory. Chiesi shall provide uniQure, promptly following uniQure's request, with any Product Data not otherwise in uniQure's possession, and uniQure shall have the right to use such Product Data in connection with development and regulatory activities conducted by or on behalf of uniQure outside the Territory.

Section 7.4 Disclosure of and License under Chiesi Improvements. Chiesi shall promptly disclose to uniQure all Improvements that are developed by Chiesi or its Affiliates (alone or in collaboration with uniQure or its Affiliates) during the Term. Chiesi shall, and hereby does, grant to uniQure a non-exclusive, worldwide, royalty-free, fully paid-up, irrevocable and perpetual (subject to Article XII) license, with the right to sublicense, under all Improvements Controlled by Chiesi and its Affiliates, to Develop, Manufacture, having Manufactured, use, and Commercialize (outside the Territory) the Product in the Field.

Section 7.5 Compliance with Third Party Agreements.

(a) The grants by uniQure under Licensed Technology set forth in Section 7.1 include the sublicense of certain Licensed Technology that is not owned by uniQure. Chiesi's rights and licenses under, or with respect to, Licensed Technology, including any Patent Prosecution or enforcement undertaken by the Parties pursuant to Article IX, are limited to the rights granted by Third Party licensors to uniQure under the Existing Third Party Licenses and are subject to all applicable restrictions, limitations and obligations imposed on uniQure or its sub-licensees in such Existing Third Party Licenses. Chiesi shall comply, and cause its Affiliates and Sub-distributors to comply, with all such restrictions, limitations and obligations *mutatis mutandis* (including Paragraphs 5.1-5.4, 8.1, 10.1, 10.2, 12.5, and 13.8-13.10 of the PHS 2011 Agreement, and Paragraphs 5.1, 5.2, 8.1, 10.1, 10.2, 12.5 and 13.6-13.8 of the PHS 2007 Agreement, a copy of which provisions is attached hereto as Exhibit B). To the extent there is a conflict between the terms of any Existing Third Party License and the rights granted to Chiesi hereunder, the terms of such Existing Third Party License shall control solely with respect to the Patent Rights and Know-How owned or controlled by the applicable Third Party licensor. Notwithstanding anything to the contrary in this Agreement, either Party may not exercise any of its rights under this Agreement (including any right to any cure period

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(including under Section 12.3) or to delay performance of an obligation (including under Article XIII or Section 15.7)) in any manner that would result in any licensor having a right to terminate an Existing Third Party License, or that would cause the other Party to be in breach of any of its obligations under any Existing Third Party License.

(b) During the Term, uniQure shall comply with the Existing Third Party Licenses in effect which are then applicable to the activities under this Agreement with respect to the Product (and in particular shall not commit any breach that would entitle the Third Party licensor to terminate such an Existing Third Party License) and shall not terminate any such Existing Third Party License without Chiesi's prior written consent. In addition, during the Term, uniQure shall promptly notify Chiesi of any written notice of breach or termination received by uniQure with respect to any such Existing Third Party License and, to the extent that uniQure does not cure such breach at least [\*\*] Business Days before the date on which the relevant licensor could terminate such Existing Third Party License due to such breach by uniQure, Chiesi shall have the right (to the extent consistent with such Existing Third Party License) to cure any such breach on uniQure's behalf and in such a case, Chiesi shall have the right to deduct (i) any and all arm's length payments made on behalf of uniQure for the above purpose, from the next due payments to be made hereunder plus (ii) interest on such payments calculated pursuant to Section 8.5 below.

(c) Any sublicensee obligations required by any Existing Third Party License to be included in a sublicense thereunder, including any required provision making the applicable Third Party licensor a third party beneficiary of any sublicense thereunder, shall be deemed to be included in this Agreement; provided that, a copy of the relevant agreement provisions has been attached hereto as Exhibit B.

(d) The license granted by uniQure in Section 7.1 with respect to the Patent Rights licensed under the Existing Third Party Licenses are subject to rights reserved by the licensors and the US government as set forth in the Existing Third Party Licenses.

Section 7.6 Additional Rights Acquired after Effective Date.

(a) During the Term, if either Party identifies the need for, or is otherwise offered, a license, covenant not to sue or similar rights to any Third Party Patent Right or Third Party Know-How that such Party in good faith believes is necessary or useful for the Development, use, Manufacture, having Manufactured, or Commercialization of the Product in the Field in the Territory ("Additional Rights"), then such Party shall promptly notify the other Party and, in any event, prior to commencing negotiation or entering into an agreement with respect to such Additional Rights, and the Parties' rights to conduct such negotiations shall be subject to the remaining provisions of this Section 7.6. The Parties shall thereafter conduct good faith discussions regarding whether such Additional Rights are necessary or useful for the Development, use, Manufacture, having Manufactured, or Commercialization of the Product in the Field in the Territory or whether they otherwise agree that such Additional Rights should be acquired.

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(b) uniQure shall have the first right (but not the obligation) to license or otherwise acquire rights to any Additional Rights. If uniQure provides written notice to Chiesi that uniQure declines to exercise such first right, then Chiesi shall have the right (but not the obligation) to pursue acquiring rights to any given Additional Rights. The Party pursuing any given Additional Rights (the "Controlling Party") shall keep the other Party (the "Non-Controlling Party") reasonably informed regarding the status thereof and shall use Commercially Reasonable Efforts to obtain from the applicable Third Party licensor the right to sublicense such Additional Rights under the licenses granted to the Non-Controlling Party hereunder.

(c) If the Controlling Party acquires rights to any Additional Rights and has the right to grant a sublicense under such Additional Rights to the Non-Controlling Party, and the Non-Controlling Party wishes to include such Additional Rights in the licenses granted to the Non-Controlling Party hereunder (or under the HemB Supply and Distribution Agreement), the Non-Controlling Party shall notify the Controlling Party of its desire to do so and the Controlling Party shall provide the Non-Controlling Party a summary of all material restrictions on the scope of the licenses granted under, and all material payment obligations that would be owed by the Non-Controlling Party with respect to, any Third Party agreement applicable to such Additional Rights. The Non-Controlling Party may, upon written notice to the Controlling Party and subject to Section 7.6(d), Section 7.6(e) and Section 7.6(f), obtain a sublicense under such Additional Rights and include such Additional Rights under the licenses granted to the Non-Controlling Party hereunder.

(d) Following such notice from the Non-Controlling Party that it desires to include any given Additional Rights under the license granted to the Non-Controlling Party hereunder (or under the HemB Supply and Distribution Agreement), (i) any such Additional Rights that do not carry financial or other obligations or restrictions shall be included automatically under the applicable license hereunder, and (ii) subject to Section 7.6(e) below, any such Additional Rights that carry financial or other obligations or restrictions shall be included under the applicable license hereunder only if the Non-Controlling Party agrees to

share the costs of such Additional Rights (including any upfront payment or similar acquisition cost to access such Additional Rights) with the Controlling Party and to assume all other obligations to, and be subject to all restrictions imposed by, the Controlling Party's licensor to the extent arising from the grant to the Non-Controlling Party under such Additional Rights (including, to the extent access to such terms have been made available to the Non-Controlling Party in unredacted form, all other terms of the Additional Rights Agreement that apply to the licenses granted to the Non-Controlling Party hereunder).

(e) If the Parties are unable, after [\*\*] Business Days, to agree as to whether any given Additional Rights are in fact necessary or useful for the Development, use, Manufacture, having Manufactured, or Commercialization of the Product in the Field in the Territory or if the Parties are unable to agree to the allocation of the costs (as specified above), then, notwithstanding Article XIII, the Parties shall jointly engage an expert panel consisting of patent attorney(s) or expert(s) in the development, manufacturing or commercialization of products comparable to the Product in question and other CMC matters, as applicable, not

regularly employed by either Party to resolve such dispute. The decision of such expert panel shall be binding on the Parties as to such dispute.

(f) Nothing in this Section 7.6 shall restrict either Party, at such Party's sole cost and expense, from licensing or otherwise acquiring any additional rights that are not necessary or useful for the Development, use, Manufacture, having Manufactured, or Commercialization of the Product in the Field in the Territory.

Section 7.7 No Implied Licenses. Except as explicitly set forth in this Agreement, neither Party grants to the other Party any license, express or implied, under its intellectual property rights.

## ARTICLE VIII FINANCIAL PROVISIONS

Section 8.1 Upfront Payment. Subject to the condition precedent pursuant to Section 12.1(b),

(a) in recognition of uniQure's past expenditure developing the Product, Chiesi shall pay uniQure a one-time, non-refundable, non-creditable fee of Five Million Euros (EUR 5,000,000);

(b) as reimbursement of uniQure's past expenditure setting up the Manufacturing site for the Product in The Netherlands, Chiesi shall pay uniQure a one-time, non-refundable, non-creditable amount of Seven Million Five Hundred Thousand Euros (EUR 7,500,000); and

(c) in consideration of uniQure making available to Chiesi Manufacturing capacity at uniQure's Manufacturing site for the Product under this Agreement, Chiesi shall pay uniQure a one-time, non-refundable, non-creditable fee of Two Million Five Hundred Thousand Euros (EUR 2,500,000);

(a) to (c), collectively, the "Upfront Payment".

Chiesi shall pay the Upfront Payment to uniQure within [\*\*] Business Days after this Agreement has become effective pursuant to Section 12.1(b), provided receipt of a proper invoice from uniQure for the Upfront Payment.

Section 8.2 Development Program Costs.

(a) Shared Costs.

(i) At the end of each calendar quarter, in accordance with paragraph (d) below, Chiesi shall pay to uniQure fifty percent (50%) of the actual Shared Costs incurred by uniQure during such calendar quarter (including, after the end of the first calendar quarter, the actual Shared Costs incurred by uniQure during the period prior to the date this Agreement has become effective pursuant to Section 12.1(b)), and uniQure shall pay to Chiesi fifty percent (50%) of the actual Shared Costs incurred by Chiesi during such calendar quarter. To this end, each Party shall provide to the other Party and to the JDC, within [\*\*] days after the end of each calendar quarter, (A) a written report with an accounting of and copies of supporting invoices for Shared Costs actually incurred by such Party during such calendar quarter

(including, after the end of the first calendar quarter, the actual Shared Costs incurred by uniQure during the period prior to the date this Agreement has become effective pursuant to Section 12.1(b)) or, with respect to Third Party invoices not timely received by a Party, for Shared Costs incurred with respect to previous calendar quarters (such amount, "uniQure Shared Cost" or "Chiesi Shared Cost", as applicable), (B) an estimate of the Shared Costs to complete the current year of the Development Program (which shall be the estimated cost to complete the current year of the Initial or newly agreed Development Plan and Budget), and (C) an estimate of the Shared Costs to complete the Development Program (which shall be the estimated cost to complete the Initial or newly agreed Development Plan and Budget).

(ii) In the case the estimated Shared Costs to complete the Development Program as set out in sub-paragraph (a)(i)(C) above is equal or within a [\*\*] percent ([\*\*]%) budget overrun of the Initial or newly agreed Development Plan and Budget, Chiesi or, as the case may be, uniQure agrees to make the quarterly payment pursuant to paragraph (i) in full. In case the estimated Shared Costs to complete the Development Program as set out in sub-paragraph (a)(i)(C) above is more than [\*\*] percent ([\*\*]%) higher than the Initial or newly agreed Development Plan and Budget, Chiesi and uniQure shall investigate the cause of this budget overrun and shall jointly agree to next steps (in the JDC). Any activity that is not budgeted in the Initial or newly agreed Development Plan and Budget shall be handled as a scope change and each scope change (incl. the budget of the scope change) must be agreed beforehand by both Parties and, when agreed, the Development Plan and Budget shall be deemed amended accordingly. Without prejudice to the foregoing provisions of this sub-paragraph (ii), any above budget overrun of the Initial or newly agreed Development Plan and Budget or any new activity not budgeted but agreed beforehand by both Parties, shall be calculated at the actual direct costs therefor (including costs for out-of-pocket expenses as well as costs for personnel at actual direct costs), without any reference to the FTE Rate.

(iii) Within [\*\*] Business Days after this Agreement has become effective pursuant to Section 12.1(b), Chiesi agrees to pay EUR [\*\*] as a first payment to prevent uniQure of pre-paying the activities of the Initial Development Plan and Budget. At the end of the Development Program,

uniQure shall credit the last quarterly payment against such EUR [\*\*] payment and pay any remaining amount, if any, to Chiesi within [\*\*] days after the end of such last calendar quarter.

(b) Chiesi Sole Costs. uniQure shall provide to Chiesi and to the JDC, within [\*\*] days after the end of each calendar quarter, a written report with an accounting of and copies of supporting invoices for Chiesi Sole Costs actually incurred by uniQure during such calendar quarter or, with respect to Third Party invoices not timely received by uniQure, for Chiesi Sole Costs incurred with respect to previous calendar quarters.

(c) uniQure Sole Costs. Chiesi shall provide to uniQure and to the JDC, within [\*\*] days after the end of each calendar quarter, a written report with an accounting of and copies of supporting invoices for uniQure Sole Costs actually incurred by Chiesi during such calendar quarter or, with respect to Third Party invoices not timely received by Chiesi, for uniQure Sole Costs incurred with respect to previous calendar quarters.

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(d) Reconciliation Payment. Within [\*\*] days after receipt of Chiesi's accountings of Chiesi Shared Cost pursuant to Section 8.2(a)(i) and of uniQure Sole Costs pursuant to Section 8.2(c), uniQure shall calculate the amount of a payment (the "Reconciliation Payment") necessary to effect the Parties' agreed allocation of costs as follows:

- (i) each Party shall bear fifty percent (50%) of all Shared Costs;
- (ii) Chiesi shall bear one hundred percent (100%) of all Chiesi Sole Costs; and
- (iii) uniQure shall bear one hundred percent (100%) of all uniQure Sole Costs;

pursuant to the following calculation:

Reconciliation Payment =

$$\begin{aligned} & (.5 \times \text{uniQure Shared Costs}) \\ & - (.5 \times \text{Chiesi Shared Costs}) \\ & + \text{Chiesi Sole Costs reported by uniQure pursuant to Section 8.2(b)} \\ & - \text{uniQure Sole Costs reported by Chiesi pursuant to Section 8.2(c)}. \end{aligned}$$

Promptly following such calculation, uniQure shall provide to Chiesi a written report thereof. Such report shall be accompanied, as applicable, by either an invoice (if the Reconciliation Payment is positive) or a credit memo (if the Reconciliation Payment is negative), which credit memo Chiesi may apply to any future payment due to uniQure under this Section 8.2. Chiesi shall pay any such invoice within [\*\*] days after receipt.

Section 8.3 Recordkeeping; Audit Rights. Each Party shall keep, and shall require its Affiliates to keep, complete and accurate records of the latest [\*\*] years of costs incurred by such Party in the conduct of Development and regulatory activities under the Development Plan and Budget (including the activities set forth in the Initial Development Plan and Budget). For the sole purpose of verifying costs included in the reports provided pursuant to Section 8.2, each Party shall have the right [\*\*] at such Party's expense to retain an independent certified public accountant selected by such Party, and reasonably acceptable to the other Party, to review such records in the location(s) where such records are maintained by the other Party or its Affiliates upon reasonable notice and during regular business hours and under obligations of confidence. Results of such review shall be made available to both Parties. If the review indicates that there was an underpayment of any amount payable to the auditing Party, the amount of such underpayment shall be remitted to the auditing Party within [\*\*] days after such review, together with interest calculated in the manner provided in Section 8.5. If the underpayment is equal to or greater than [\*\*] percent ([\*\*]%) of the amount that was otherwise due, the audited Party shall pay all of the auditing Party's reasonable out-of-pocket expenses of such review. If the review indicates that there was an overpayment of any amounts by the audited Party,

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the audited Party may apply the amount of such overpayment to any future payment due to the auditing Party under Section 8.2.

Section 8.4 Method of Payment. All amounts payable by a Party (the "Paying Party") hereunder shall be paid by or on behalf of such Paying Party in Euros. Shared Costs, uniQure Sole Costs or Chiesi Sole Costs incurred in a currency other than Euros shall be expressed in their Euro equivalent, calculated on the last Business Day of the calendar quarter to which the applicable report relates using the currency converter at [www.oanda.com](http://www.oanda.com). All payments due to a Party (the "Receiving Party") hereunder shall be made by wire transfer directly to an account designated by the Receiving Party in writing. The Receiving Party shall be responsible for all charges from the receiving bank due to the receipt of the wire transfer. The Paying Party shall be responsible for all other bank costs.

Section 8.5 Late Payments. Any payment under this Agreement that is not paid on or before the date such payment is due shall bear interest at the lesser of (a) [\*\*] per year, or (b) the highest rate permitted by Applicable Laws, calculated on the number of days such payments are overdue.

Section 8.6 Tax Withholding. To the extent that any payments hereunder by the Paying Party to the Receiving Party are subject to tax, the Paying Party shall pay such tax; provided, however, that, with respect to any payments subject to withholding tax, the Paying Party shall pay the applicable withholding tax amount to the relevant taxing authority and promptly provide the Receiving Party with all necessary documentation for the Receiving Party to recover such tax. The Paying Party will take all reasonable and lawful steps to minimize the amount of any such withholding tax obligation and the Receiving Party shall promptly provide all information and documentation in its possession necessary for doing so.

Section 8.7 Blocked Payments. In the event that, by reason of Applicable Laws in any country, it becomes impossible or illegal for the Paying Party to transfer payments to the Receiving Party, the Paying Party shall, to the extent consistent with Applicable Laws, have such royalties or other payments paid to the Receiving Party by an Affiliate of the Paying Party. To the extent such payment is not consistent with Applicable Laws, the Paying Party shall deposit such payments in local currency in the relevant country to the credit of the Receiving Party in a recognized banking institution designated by the Receiving Party or, if none is designated by the Receiving Party within a period of [\*\*] days after written request from the Paying Party, in a recognized banking institution selected by the Paying Party and identified in a notice in writing given to the Receiving Party.

(a) Existing Intellectual Property. Except as expressly set forth in this Agreement and subject to the licenses granted under this Agreement, as between the Parties each Party shall retain all right, title and interest in and to the Patent Rights, Know-How and other intellectual property rights owned by or Controlled by such Party or its Affiliates as of the Effective Date.

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(b) Solely Owned Know-How. Except as expressly set forth in this Agreement and subject to the licenses granted by such Party under this Agreement, as between the Parties each Party or its Affiliate, as applicable, shall exclusively own all right, title and interest in and to all Know-How made or conceived solely by the employees, agents or consultants of such Party or its Affiliates in the course of performing its activities under this Agreement.

(c) Joint Know-How. All Know-How made or conceived jointly by employees, agents or consultants of uniQure or its Affiliates, on the one hand, and employees, agents or consultants of Chiesi or its Affiliates, on the other hand, shall be owned jointly on the basis of each Party having an undivided one-half (½) interest in the whole ("Joint Know-How"), and each Party hereby assigns to the other Party a sufficient interest in its rights in and to the Joint Know-How so as to effect such joint ownership. Subject to the licenses granted herein and each Party's payment obligations hereunder, each Party shall have the right to exploit the Joint Know-How, or sell, license or otherwise transfer or grant rights under Joint Know-How, or any Joint Patents directed to the Joint Know-How, to its Affiliates or any Third Party, without any duty to account to the other Party; provided that, during the Term neither Party nor its Affiliates may use, sell, license or otherwise transfer or grant rights under Joint Know-How, or any Joint Patents directed to such Joint Know-How, to any Affiliate or Third Party in any manner which would conflict with, or limit the scope of, any of the rights or licenses granted to the other Party hereunder.

(d) Inventorship. For purposes of determining the Parties' rights under this Agreement, the determination of inventorship shall be made in accordance with US patent laws.

Section 9.2 Prosecution and Maintenance of Patent Rights.

(a) Licensed Patents. Subject to any rights of and obligations to uniQure's Third Party licensors, uniQure shall have the exclusive right and obligation to conduct Patent Prosecution for the Licensed Patents (other than any Joint Patent) in the Territory, in uniQure's name, and to control any interference, derivation proceeding, reexamination, review, opposition and similar proceedings relating thereto. Subject to any rights of and obligations to uniQure's Third Party licensors, uniQure shall promptly and regularly inform and consult with Chiesi regarding the Patent Prosecution, including any interference, derivation proceeding, reexamination, review, opposition and similar proceedings relating thereto, of all Licensed Patents in the Territory.

(b) Chiesi Patents. Chiesi shall have the exclusive right and obligation to conduct Patent Prosecution for the Chiesi Patents (other than any Joint Patents) in Chiesi's name, and to control any interference, derivation proceeding, reexamination, review, opposition and similar proceedings relating thereto. Chiesi shall promptly and regularly inform and consult with uniQure regarding the Patent Prosecution, including any interference, derivation proceeding, reexamination, review, opposition and similar proceedings relating thereto, of all Chiesi Patents.

(c) Joint Patents. uniQure shall have the first right and option (but not the obligation) to conduct Patent Prosecution for the Joint Patents in uniQure's name, and to control any interference, derivation proceeding, reexamination, review,

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opposition and similar proceedings relating thereto. In the event that uniQure elects to conduct Patent Prosecution according to the foregoing sentence, uniQure shall grant, and hereby grants, to Chiesi, subject to the terms and conditions of this Agreement, a non-exclusive, worldwide, royalty-free, fully paid-up, irrevocable, perpetual license, with the right to grant sublicenses, under the Joint Patents to Develop, use, Manufacture, have Manufactured, and Commercialize the Product in the Field. In the event that uniQure elects not to conduct Patent Prosecution for, or elects to abandon, any Joint Patent, or declines to control any related interference, derivation proceeding, reexamination, review, opposition or similar proceedings, uniQure shall give Chiesi reasonable written notice to this effect, sufficiently in advance to permit Chiesi, in its sole discretion, to undertake such Patent Prosecution, or to control such interference, derivation proceeding, reexamination, review, opposition or similar proceedings, without a loss of rights, and thereafter Chiesi may, upon written notice to uniQure and in Chiesi's name, conduct Patent Prosecution for such Joint Patents and control such interference, derivation proceeding, reexamination, review, opposition or similar proceedings. If required under Applicable Laws in order for the prosecuting Party to control any interference, derivation proceeding, reexamination, review, opposition and similar proceedings relating to any Joint Patent, the other Party shall join as a party to such interference, derivation proceeding, reexamination, review, opposition and similar proceedings.

(d) Cooperation. Each Party agrees to cooperate with the other Party with respect to Patent Prosecution, including any interference, derivation proceeding, reexamination, review, opposition and similar proceedings relating thereto, of Joint Patents pursuant to Section 9.2(c), subject to any rights of, and obligations to, uniQure's Third Party licensors, including by:

(i) executing all such documents and instruments and performing of such acts as may be reasonably necessary in order to permit the other Party to continue any Patent Prosecution that such Party has elected not to pursue, as provided for in Section 9.2(c);

(ii) making its employees, agents and consultants reasonably available to the other Party (or to the other Party's authorized attorneys, agents or representatives), to the extent reasonably necessary to enable the prosecuting Party to undertake Patent Prosecution;

(iii) providing (itself or through patent counsel) the other Party with a copy of each proposed material correspondence pertaining to substantive Patent Prosecution on the merits with World Intellectual Property Office ("WIPO") or the European Patent Office ("EPO"), as well as providing draft copies of patent applications to be submitted to the WIPO under the Patent Cooperation Treaty, or submitted to any patent office in the Territory in a form substantially different from that previously submitted to the WIPO, reasonably in advance of any applicable filing or response deadline to allow the other Party to review and comment on the content of such proposed correspondence and advise the prosecuting Party as to the conduct of such Patent Prosecution, which comments and advice the prosecuting Party will consider in good faith;

(iv) providing (itself or through patent counsel) the other Party with copies of all material correspondence pertaining to substantive Patent

Prosecution on the merits with the WIPO or the EPO after its submission or receipt, as the case may be; and

- (v) seeking patent term extensions, adjustments, and the like wherever available for the Product.

### Section 9.3 Third Party Infringement.

(a) Notice. Each Party shall promptly report in writing to the other Party during the Term any known or suspected infringement of any Valid Claims within the Licensed Patents, Chiesi Patents or Joint Patents involving the use, manufacture or commercialization of a product or product candidate that is or would likely be a Competing Product ("Competitive Infringement"), and shall provide the other Party with all available evidence supporting such infringement or suspected infringement. Promptly after receipt of a notice of a Competitive Infringement in the Territory, the Parties shall discuss in good faith the infringement and appropriate actions that could be taken to cause such Competitive Infringement to cease.

(b) Enforcement of Patents. The Party with responsibility under Section 9.2 for Patent Prosecution of the Patent Right that is subject of the Competitive Infringement shall have the exclusive right to initiate a suit or take other appropriate action that it believes is reasonably required to prevent or abate actual or threatened infringement of, or otherwise enforce, in the best commercial interests of the Product, the applicable Patent Right. The Party filing any such suit or taking any such action shall control all decision making related to any such suit or action, subject to Section 9.3(c) below.

(c) Conduct of Actions. The Party initiating suit or action pursuant to Section 9.3(b) with respect to Competitive Infringement in the Territory shall have the sole and exclusive right to select counsel for such suit or action. At the initiating Party's request and expense, the other Party shall join as a party to the suit or action. Such other Party shall offer reasonable assistance to the initiating Party in connection therewith. The initiating Party shall provide the other Party with an opportunity to make suggestions and comments regarding such suit or action. The initiating Party shall, to the extent permitted by Applicable Laws, keep the other Party promptly informed, and shall from time to time consult with such other Party, regarding the status of any such suit or action and shall provide such other Party with copies of all material documents (including complaints, answers, counterclaims, material motions, orders of the court, memoranda of law and legal briefs, interrogatory responses, depositions, material pre-trial filings, expert reports, affidavits filed in court, transcripts of hearings and trial testimony, trial exhibits and notices of appeal) filed in, or otherwise directly relating to, such suit or action. The Party not initiating such suit or action shall have the right to participate and be represented in any such suit by its own counsel at its own expense. Neither Party shall conduct any such suit or action in a manner that materially places at risk the scope or validity of any Joint Patent, and neither Party shall settle or compromise any claim or proceeding relating to any Joint Patent, without obtaining the prior written consent of the other Party. If Chiesi or any of its Affiliates conduct any such suit or action in a manner that materially places at risk the scope or validity of any Licensed Patent, uniQure may terminate this Agreement in accordance with the provisions of Section 12.4. If uniQure or any of its Affiliates conduct any such suit or action in a manner that materially places at risk the

scope or validity of any Chiesi Patent, Chiesi may terminate this Agreement in accordance with the provisions of Section 12.4.

(d) Recoveries. With respect to any suit or action to protect any Joint Patent referred to in Section 9.3(b) above, any recovery obtained by a Party as a result of any such proceeding, by settlement or otherwise, shall be applied in the following order of priority:

- (i) first, such Party shall pay to the applicable licensor any amount to which such licensor is entitled pursuant to the terms of any Existing Third Party License or Additional Rights Agreement; and
- (ii) second, any remainder shall be allocated equally between the Parties.

Section 9.4 Claimed Infringement. In the event that a Party becomes aware of any claim or threat of claim that the Development, use, Manufacture, have Manufactured or Commercialization hereunder of the Product infringes or misappropriates the intellectual property rights of any Third Party, such Party shall promptly notify the other Party. Each Party shall provide to the other Party copies of any notices such Party receives from Third Parties regarding any alleged infringement of Third Party Patent Rights or any alleged misappropriation of Third Party Know-How. Such notices shall be provided promptly, but in no event after more than [\*\*] days following receipt thereof. In any such instance, the Parties shall cooperate in undertaking an appropriate course of action.

Section 9.5 Patent Invalidity Claim. If a Third Party at any time asserts a claim that any Licensed Patent (including a Joint Patent) or Chiesi Patent (including a Joint Patent) that covers the composition of matter of the Product or the method of use of the Product in the Field in the Territory is invalid or otherwise unenforceable ("Invalidity Claim"), either as a defense in an infringement action brought by Chiesi or uniQure pursuant to Section 9.3 or in an action brought against Chiesi or uniQure under Section 9.4, including any declaratory judgment action, the Parties shall cooperate with each other in (i) preparing and formulating a response to such Invalidity Claim and (ii) undertaking any further and appropriate course of action. Neither Party shall settle or compromise any Invalidity Claim without the consent of the other Party, which consent shall not be unreasonably withheld.

Section 9.6 Licensor Rights. All obligations under Sections 9.2 through 9.5 are subject to the rights of the relevant licensor pursuant to the terms of any Existing Third Party License or Additional Rights Agreement.

## ARTICLE X CONFIDENTIALITY

Section 10.1 Confidential Information. All Confidential Information disclosed by a Party or any of its Affiliates to the other Party or any of its Affiliates before or during the Term shall not be used by the receiving Party or any of its Affiliates except in connection with the activities contemplated by this Agreement, shall be maintained in confidence by the receiving Party and its Affiliates, and shall not otherwise be disclosed by the receiving Party or its Affiliates to any Third Party

(except as set forth in the remainder of this Article X), without the prior written consent of the disclosing Party, except to the extent that the Confidential Information:

(a) was known or used by the receiving Party or any of its Affiliates prior to its date of disclosure by the disclosing Party;

(b) either before or after the date of the disclosure to the receiving Party hereunder or under the Confidentiality Agreement is lawfully disclosed to the receiving Party or any of its Affiliates by a Third Party rightfully in possession of and with the right to disclose such Confidential Information other than under an obligation of confidentiality;

(c) either before or after the date of the disclosure to the receiving Party hereunder or under the Confidentiality Agreement becomes generally known to the public through no fault or omission on the part of the receiving Party or its Affiliates;

(d) is independently developed by or for the receiving Party or any of its Affiliates without reference to or reliance upon any of the other Party's Confidential Information; or

(e) is required to be disclosed by the receiving Party or its Affiliates to comply with Applicable Laws, which may include the rules of Euronext, of the US Securities and Exchange Commission ("SEC"), or of any other stock exchange, or to defend or prosecute litigation or arbitration or to comply with legal process; provided that, the receiving Party provides prior written notice of such disclosure to the disclosing Party (to the extent feasible) and only discloses Confidential Information of the other Party to the extent necessary for such legal compliance or litigation purpose; and provided, further, that such information shall otherwise remain Confidential Information (subject to the exceptions in this Section 10.1).

Notwithstanding the foregoing, clauses (a), (b) and (d) shall not alter the requirement to keep the terms and conditions of this Agreement confidential, as set forth herein, subject to the remainder of this Article X.

Section 10.2 Employee, Director, Consultant and Advisor Obligations. Chiesi and uniQure each agrees that it and its Affiliates shall provide Confidential Information received from the other Party only to the receiving Party's employees, directors, consultants, agents and advisors, and to the employees, directors, consultants, agents and advisors of the receiving Party's Affiliates, who have a need to know such Confidential Information to assist the receiving Party in fulfilling its obligations under this Agreement and who are bound by obligations of confidentiality and non-use that are at least as restrictive as those set forth in this Agreement. Each Party shall remain responsible for any failure by any of its or its Affiliates' employees, directors, consultants, agents and advisors to treat such Confidential Information as required under this Article X.

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Section 10.3 Publicity.

(a) Following execution of this Agreement, the Parties shall jointly or separately issue a press release, in a text to be agreed upon between the Parties in advance, announcing the execution of this Agreement and the Commercialization Agreement.

(b) Thereafter, each Party shall only issue press releases (other than the press release pursuant to paragraph (a) above) or make other public disclosures regarding this Agreement or the Parties' activities under this Agreement (each such press release or public disclosure, a "Subject Disclosure");

(i) that have been approved in writing in advance by the other Party (such approval not to be unreasonably withheld, conditioned or delayed);

(ii) if advised by counsel to issue such Subject Disclosure in order to comply with Applicable Laws, which may include the disclosure rules of SEC or a similar regulatory agency in a country in the Territory or of Euronext or any other stock exchange of other securities trading institution (for clarity such issuance is also subject to Section 10.3(c));

(iii) subject to Section 10.3(c), if the contents of such Subject Disclosure have previously been made public other than through a breach of this Article X by a Party; or

(iv) subject to sub-paragraph (i) above, to the extent that such Subject Disclosure describes one or more of the following:

(A) preclinical results with respect to the Product;

(B) the commencement, completion or "top-line" results of clinical studies of the Product;

(C) the completion of patient enrollments for clinical studies of the Product;

(D) the filing for or receipt of Marketing Authorization with respect to the Product;

(E) the Patent Prosecution or enforcement of any of the Licensed Patents, including the issuance of any patent included in the Licensed Patents;

(F) the receipt of any regulatory exclusivity for the Product; or

(G) the first Party's presence or participation at scientific, financial or investor forums.

(c) Unless not feasible under the circumstances because of the need to comply with Applicable Laws or stock exchange rules, the Party making a Subject Disclosure shall provide the other Party with a draft Subject Disclosure at

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least [\*\*] Business Days prior to its intended publication for the other Party's review. Such other Party may provide the first Party with suggested modifications to the draft Subject Disclosure. The first Party shall consider in good faith the other Party's suggestions in issuing such Subject Disclosure.

(d) For clarity, nothing in this Agreement shall restrict each Party from issuing press releases or making other public disclosures regarding such Party's development, manufacturing or commercialization activities with respect to any product other than the Product, or, with reference to uniQure only, with respect to any Product outside the Territory.

Section 10.4 Other Disclosures. Notwithstanding anything in this Agreement to the contrary, each Party shall have the right to disclose the other Party's Confidential Information (including the terms of this Agreement) (as applicable):

(a) to such Party's then-current or potential investors, lenders, acquirers, investment bankers, and other Third Parties in connection with financing, partnering (to the extent consistent with this Agreement) and acquisition activities, solely on a need-to-know basis and under obligations of confidentiality and non-use that are at least as restrictive as those set forth in this Article X;

(b) as required by the Existing Third Party Licenses or any Additional Rights Agreement;

(c) to conduct Patent Prosecution or enforcement of Patent Rights for which such Party is responsible hereunder;

(d) to such Party's then-current or potential collaborators, and Third Party contractors (including contract manufacturers and Sub-distributors) for purposes of engaging in the Development, use, Manufacture or Commercialization of the Product as contemplated hereunder, solely on a need-to-know basis and under obligations of confidentiality and non-use that are at least as restrictive as those set forth in this Article X.

Section 10.5 Publications.

(a) Notwithstanding Section 10.3 and Section 10.4, a Party (the "Publishing Party") which is, or whose Affiliates is, seeking to publish or publicly present scientific or technical data, results or other information with respect to the Product shall provide the other Party and the JDC with a copy of any proposed publication or presentation at least [\*\*] days (or at least [\*\*] days in the case of abstracts or oral public presentations) prior to submission for publication or presentation so as to provide such other Party with an opportunity to recommend any changes it reasonably believes are necessary to continue to maintain such other Party's Confidential Information in accordance with the requirements of this Agreement or to not jeopardize the patentability of any results or data.

(b) If the non-Publishing Party notifies the Publishing Party that such publication or presentation, in the non-Publishing Party's reasonable judgment, (i) discloses an invention for which the non-Publishing Party desires to seek patent protection, or (ii) contains any Confidential Information of the non-Publishing Party,

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or could be expected to have an adverse effect on the commercial value of any Confidential Information disclosed by the non-Publishing Party to the Publishing Party, the Publishing Party shall delete such Confidential Information from the proposed publication or presentation and shall further delay such publication or presentation for a period reasonably sufficient to permit the timely preparation and filing of a patent application(s) on any invention disclosed in such publication or presentation (but no more than [\*\*] days from the date of the non-Publishing Party's notice thereof).

Section 10.6 Use of Names.

(a) Chiesi, its Affiliates and Third Party contractors shall not use the name "St. Jude Children's Research Hospital" or any variation of that name, or any trademarks or logos belonging to St. Jude, or the names of any of St. Jude's trustees, officers, faculty, student, employees, or agents, or any adaptation of such names, or any term of the St. Jude Agreement in any promotional material or other public announcement or disclosure or in connection with the Commercialization of the Product, without the prior written approval of St. Jude; except (i) in annual reports or as part of required regulatory or financial disclosures to the FDA, SEC or other US federal or foreign agencies; and (ii) where otherwise required by Applicable Laws, provided that, Chiesi shall notify St. Jude in advance of any disclosure to be made under these exceptions.

(b) Chiesi, its Affiliates and Third Party contractors shall not state or imply that the PHS Agreements are an endorsement by the US government, PHS, any other US government organizational unit, or any US government employee. Additionally, Chiesi and its Affiliates shall not use the names of NIH, FDA, PHS, or HHS or the US government or their employees in any advertising, promotional, or sales literature without the prior written approval of PHS.

Section 10.7 Term. All obligations of confidentiality imposed under this Article X shall expire [\*\*] years following termination or expiration of this Agreement, except to the extent any Existing Third Party License or Additional Rights Agreement extends such obligations; provided, however, that the receiving Party shall maintain the confidentiality of any of the other Party's trade secrets indefinitely until such trade secret is no longer a trade secret.

## ARTICLE XI REPRESENTATIONS, WARRANTIES AND COVENANTS

Section 11.1 Representations and Warranties of Both Parties. Each Party hereby represents and warrants to the other Party, as of the Effective Date, that:

(a) such Party is duly organized, validly existing and in good standing under the Applicable Laws of the jurisdiction of its incorporation and has full corporate power and authority to enter into this Agreement and to carry out the provisions hereof;

(b) such Party has taken all necessary action on its part to authorize the execution and delivery of this Agreement and the performance of its obligations hereunder;

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(c) this Agreement has been duly executed and delivered on behalf of such Party, and constitutes a legal, valid, binding obligation, enforceable against it in accordance with the terms hereof; and

(d) the execution, delivery and performance of this Agreement by such Party does not conflict with any agreement, instrument or binding understanding, oral or written, to which it is a party or by which it is bound, nor violate any Applicable Law of any court, governmental body or administrative or other agency having jurisdiction over such Party.

Section 11.2 Representations and Warranties of uniQure. uniQure hereby represents and warrants to Chiesi, as of the Effective Date that:

(a) Exhibit E attached hereto is a complete and correct list of all Licensed Patents that claim the composition of matter, or method of use or manufacture, of the Product and are Controlled by uniQure and for which uniQure controls Patent Prosecution as of the Effective Date;

(b) uniQure Controls the Licensed Technology and has the full right, power and authority to grant all rights and licenses to Chiesi with respect to the Licensed Technology under this Agreement;

(c) To uniQure's knowledge, it has not (i) employed or used any contractor or consultant that employs any individual or entity debarred by the FDA (or subject to a similar sanction of EMA), or (ii) employed any individual or entity that is the subject of an FDA debarment investigation or proceeding (or similar proceeding of EMA), in each of clauses (i) and (ii), in the conduct of development activities directed to the Product;

(d) To uniQure's knowledge, the Development and Commercialization of the Product in the Territory, as anticipated hereunder, does not infringe upon any intellectual property rights of any Third Party

(e) uniQure has not received any written allegation from a Third Party that any of the issued Licensed Patents is invalid or unenforceable and, except as disclosed in Exhibit H, to uniQure's knowledge, none of such Licensed Patents is infringed by any Third Party;

(f) uniQure has not received, with respect to the Product as Developed by uniQure, any written notice from a Third Party claiming infringement or misappropriation of any Patent Right or any Know-How owned by such Third Party; and

(g) uniQure has provided Chiesi with a complete and correct copy of each of the Existing Third Party Licenses.

Section 11.3 Representation and Warranty of Chiesi. Chiesi hereby represents and warrants to uniQure, as of the Effective Date, that Chiesi Controls the Chiesi Technology and has the full right, power and authority to grant all rights and licenses to uniQure under this Agreement.

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Section 11.4 Mutual Covenants. Each Party hereby covenants to the other Party that:

(a) All employees of such Party or its Affiliates working under this Agreement are and will be under the obligation to assign all right, title and interest in and to their inventions and discoveries arising in the performance of such work, whether or not patentable, to (i) such Party as the sole owner thereof or (ii) to one of such Party's Affiliates as the sole owner thereof so that such Party Controls such inventions and discoveries;

(b) To its knowledge, such Party will not, in the conduct of its activities under this Agreement, (i) employ or use any contractor or consultant that employs any individual or entity debarred by the FDA (or subject to a similar sanction of EMA), or (ii) employ any individual who or entity that is the subject of an FDA debarment investigation or proceeding (or similar proceeding of EMA), in each of clauses (i) and (ii) in the conduct of its activities under this Agreement;

(c) Such Party shall perform its activities pursuant to this Agreement in compliance in all material respects with Applicable Laws; and

(d) Neither Party shall, during the Term, grant any right or license to any Third Party relating to any of the intellectual property rights it Controls which would conflict with, or limit the scope of, any of the rights or licenses granted or to be granted to the other Party hereunder.

Section 11.5 DISCLAIMER. EXCEPT AS OTHERWISE EXPRESSLY SET FORTH IN THIS AGREEMENT, NEITHER PARTY MAKES ANY REPRESENTATION OR EXTENDS ANY WARRANTY OF ANY KIND, EITHER EXPRESS OR IMPLIED, INCLUDING ANY WARRANTY THAT ANY PATENT RIGHTS ARE VALID OR ENFORCEABLE, AND EXPRESSLY DISCLAIMS ALL IMPLIED WARRANTIES OF MERCHANTABILITY, FITNESS FOR A PARTICULAR PURPOSE AND NON-INFRINGEMENT. WITHOUT LIMITING THE GENERALITY OF THE FOREGOING, EACH PARTY DISCLAIMS ANY WARRANTIES WITH REGARDS TO: (A) THE SUCCESS OF ANY STUDY OR CLINICAL TRIAL COMMENCED UNDER THIS AGREEMENT; (B) THE SAFETY, USEFULNESS FOR ANY PURPOSE OR NON-INFRINGEMENT OF ANY PRODUCT; OR (C) THE VALIDITY, ENFORCEABILITY OR NON-INFRINGEMENT OF ANY INTELLECTUAL PROPERTY RIGHTS OR TECHNOLOGY IT PROVIDES OR LICENSES TO THE OTHER PARTY UNDER THIS AGREEMENT.

## ARTICLE XII TERM AND TERMINATION

Section 12.1 Term.

(a) General. This Agreement shall become effective as of the Effective Date and shall remain in force, on a country-by-country basis, for the longer of (i) twelve (12) years from the First Commercial Sale of the Product in the relevant country of the Territory; (ii) expiry of any regulatory exclusivity granted by any Marketing Authorization or any other Regulatory Approval in the relevant country of

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the Territory; or (iii) expiry of the last Valid Claim Covering the Product in the relevant country of the Territory. Unless terminated by a Party with three (3) months written notice to the other Party to the end of the above initial or any subsequent term, this Agreement shall automatically be renewed for successive five (5) year terms (the initial and each subsequent term, the "Term").

(b) Condition Precedent. This Agreement, and any ancillary agreement concluded between the Parties in connection herewith, including the SDEA, and the Commercialization Agreement and the agreement regarding the equity investment of Chiesi in uniQure concluded on the date hereof, shall become effective once the Parties have received consent from PHS as the Third Party licensor to the subcontracting of the rights and licenses licensed by uniQure as licensee under the PHS Agreements to Chiesi. uniQure shall use Commercially Reasonable Efforts to obtain such consent on or prior to [\*\*]. If, despite uniQure's Commercially Reasonable Efforts, such consent has not been obtained from PHS by the end of [\*\*], this Agreement and all other agreements that are subject to the condition precedent pursuant to sentence 1 shall be deemed null and void as of the Effective Date, unless, prior to the end of such period, following a corresponding request of either Party, the Parties mutually agree in writing on an extension of such period. The Parties agree that (i) costs and expenses incurred in connection with the preparation and execution of this Agreement as well as obtaining of the aforementioned consent shall not be reimbursed, provided, however, that uniQure shall pay back to Chiesi any payments received in connection with this Agreement on or prior to [\*\*] (or such extended period mutually agreed between the Parties in accordance with the foregoing), and (ii) Sections 10.1, 10.2 and 10.7 shall apply *mutatis mutandis*.

Section 12.2 Termination for Convenience. Chiesi may terminate this Agreement for convenience upon six (6) months' prior written notice to uniQure at any time during the Term, following the first six (6) months of the Agreement.

Section 12.3 Termination for Material Breach. Upon any material breach of this Agreement by either Party (in such capacity, the "Breaching Party"), the other Party (in such capacity, the "Non-Breaching Party") may terminate this Agreement by providing [\*\*] days' prior written notice to the Breaching Party, specifying the material breach. The termination shall become effective at the end of the [\*\*] day period unless the Breaching Party cures such breach during such [\*\*] day period.

In the event that either Party is the Breaching Party or that either Party, its Affiliates or Third Party contractors breach any of the requirements under any Existing Third Party Licenses or Additional Rights Agreements, such Party's cure period described in the preceding paragraph shall be eliminated or reduced to the extent necessary to prevent the breach from giving a licensor a right to terminate an Existing Third Party License or any Additional Rights Agreement or from causing the other Party to be in breach of its obligations under any Existing Third Party License or any Additional Rights Agreement.

Section 12.4 Termination for Patent Challenge. If either Party or any of its Affiliates or Third Party contractors challenges the validity, enforceability, patentability or scope of any claim included in any Patent, (any of the foregoing, a "Patent Challenge"), the other Party shall have the right to terminate this Agreement immediately upon written notice to such Party.

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Section 12.5 Effects of Termination by Chiesi for Convenience or by uniQure for Chiesi Uncured Breach or Patent Challenge. Upon termination of this Agreement by Chiesi in its entirety pursuant to Section 12.2 (Termination for Convenience) or by uniQure pursuant to Section 12.3 (Termination for Material Breach) or pursuant to Section 12.4 (Termination for Patent Challenge):

- (a) All rights and licenses granted by uniQure to Chiesi shall terminate and revert to uniQure, and all rights and licenses granted by Chiesi to uniQure shall survive and become fully paid-up, irrevocable and perpetual;
- (b) Chiesi shall promptly provide to uniQure a fair and accurate description of the status of its Development Program activities through the effective date of termination;
- (c) Chiesi shall promptly assign to uniQure the entire right, title, and interest in and to, and transfer to uniQure all copies of, any Product Data in Chiesi's or its Affiliates' or Third Party contractors' possession or control;
- (d) At uniQure's option and upon uniQure's request as to any or all of the following (in whole or in part), Chiesi (or its relevant Affiliate) shall promptly:
  - (i) assign or cause the assignment to uniQure of any and all applicable Third Party agreements for the Product, including agreements with contract research organizations and other agreements relating to the Development of the Product, in each case to the extent assignable; provided, however, that, to the extent such agreements are not specific to the Product, or are not assignable, Chiesi shall, at uniQure's request, hold such agreements for the benefit of uniQure and Chiesi and its Affiliates shall take such actions as uniQure may reasonably request so as to provide uniQure with the benefits thereunder with respect to the Product;
  - (ii) Chiesi and its Affiliates shall waive any obligations of confidentiality, non-competition and exclusivity imposed on its Third Party service providers, in order to permit uniQure to negotiate agreements with such service providers to develop, import, export and use the Product; and
  - (iii) at uniQure's request, on a clinical trial-by-clinical trial basis with respect to any on-going clinical trial with the Product being conducted by or under authority of Chiesi or its Affiliates as of the date of the termination notice, either:
    - (A) terminate any such clinical trial as of the date of the termination notice in a manner conforming to Applicable Laws and provide all related data to uniQure promptly following termination of such clinical trial,
    - (B) continue to conduct such clinical trial to completion, keeping uniQure fully informed of the status of such clinical trial, and provide all related data to uniQure promptly following its completion, or
    - (C) promptly transfer such clinical trial to uniQure or its designee and continue to conduct such clinical trial up to completion of such

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transfer, keeping uniQure fully informed of the status of such clinical trial, and provide all related data to uniQure promptly following completion of such transfer;

(e) In accordance with uniQure's request, Chiesi shall promptly return to uniQure, or promptly destroy and certify to uniQure in writing that it has destroyed, all materials and records in its possession or Control containing Confidential Information of uniQure, except for a single copy of such

(f) With respect to Chiesi Patents, (i) the provisions of Section 9.2(b), Section 9.3(b) and Section 9.5 shall apply to the Chiesi Patents with the respective roles of the Parties reversed, and (ii) if uniQure initiates suit pursuant to Section 9.3(b), uniQure may retain any damages, settlements, accounts of profits, or other financial compensation recovered from a Third Party based upon such suit;

(g) uniQure shall have the sole right to conduct Patent Prosecution with respect to the Joint Patents, notwithstanding Section 9.2(c), and to enforce the Joint Patents, notwithstanding Section 9.3;

(h) Neither Chiesi nor any of its Affiliates shall use or grant rights to any Affiliate or Third Party for Joint Know-How in relation to any Competing Product during the [\*\*] following termination;

(i) The restrictions on Chiesi and its Affiliates set forth in Section 6.1 shall survive for [\*\*] following termination; and

(j) Chiesi shall execute all documents and take all such further actions as may be reasonably requested by uniQure in order to give effect to the foregoing clauses (a) through (i).

Section 12.6 Effects of Termination by Chiesi for uniQure Uncured Breach or Patent Challenge. Upon termination of this Agreement by Chiesi pursuant to Section 12.3 (Termination for Material Breach) or pursuant to Section 12.4 (Termination for Patent Challenge), the consequences set forth in Section 12.5 shall apply, *mutatis mutandis*; provided that, the Parties shall negotiate in good faith appropriate consideration payable by uniQure to Chiesi in connection therewith reflecting the stage to which the Parties have Developed the Product prior to such termination.

Section 12.7 Upon expiration of the Term with respect to this Agreement pursuant to Section 12.1:

(a) all rights, privileges and licenses granted hereunder to Chiesi shall become fully paid-up, irrevocable and perpetual;

(b) all rights, privileges and licenses granted hereunder to uniQure shall become fully paid-up, irrevocable and perpetual;

(c) at any time upon written request of the disclosing Party, unless expressly set forth otherwise in this Agreement, the receiving Party shall cease use of and return or at the disclosing Party's request destroy all Confidential Information of

the disclosing Party and all copies thereof except for a single copy of such Confidential Information that may be retained confidentially for legal purposes only.

Section 12.8 Survival.

(a) Upon expiration or termination of this Agreement for any reason, all rights and obligations of each Party shall terminate hereunder, except as expressly set forth in Section 12.5, Section 12.6, Section 12.7 or this Section 12.8; provided, however, that nothing in this Agreement shall be construed to release either Party from any obligations or liabilities that matured prior to the effective date of expiration or termination, or which are attributable to a period prior to such expiration or termination.

(b) Notwithstanding anything in this Agreement to the contrary, the following provisions shall expressly survive any expiration or termination of this Agreement in accordance with their terms: Article I, Section 6.2, Section 7.3, Article VIII (in each case, to the extent any amounts are due but unpaid as of the effective date of expiration or termination or thereafter pursuant to Section 12.5); Section 9.1; Article X; Section 11.5; Section 12.5; Section 12.6; Section 12.7; Section 12.8; Article XIII; Article XIV; and Article XV.

(c) Termination of this Agreement shall be in addition to, and shall not prejudice, the Parties' remedies at law or in equity, including the Parties' ability to receive legal damages or equitable relief with respect to any breach of this Agreement, regardless of whether or not such breach was the reason for the termination.

### ARTICLE XIII DISPUTE RESOLUTION

Section 13.1 Resolution of Certain Disputes Other Than by Arbitration.

(a) Either Party's exercise of its right to terminate this Agreement for the other Party's material breach in accordance with Section 12.3, to the extent necessary to prevent such breach (whether by the other Party or its Affiliate) or from giving a licensor a right to terminate an Existing Third Party License or an Additional Rights Agreement or from causing the first Party to be in breach of its obligations under any Existing Third Party License or any Additional Rights Agreement, (a "Non-Arbitrable Termination Dispute") shall not be subject to the dispute resolution procedures of Article II or Section 13.2 prior to termination. The other Party shall not be entitled to injunctive relief to prevent or delay such termination, and shall only be entitled to monetary damages in the event that it thereafter disputes such termination pursuant to Section 13.2(b)(i) and the arbitrators determine that the first Party has not properly exercised its termination right hereunder.

(b) Any dispute that could be resolved by the JSC under Section 2.1(e), if unresolved by the JSC, shall be finally resolved as set forth in Section 13.2.

Section 13.2 Resolution of Other Disputes by Executive Officers and Arbitration.

(a) With the exception of Non-Arbitrable Termination Disputes, in the event any dispute arises out of or in relation to or in connection with any of the Collaboration Agreements, including any issue relating to the interpretation or application of the Collaboration Agreements, the Parties shall use good faith efforts to resolve such dispute within [\*\*] days, through the JSC if the dispute is within the responsibilities of the JSC, or, if the dispute is not within the

responsibilities of the JSC, through informal negotiations between the respective representatives of the Parties. If the JSC or the applicable representatives are unable to resolve such dispute within such [\*\*] day period, the Parties shall refer such dispute to the Executive Officers for resolution. If a dispute is referred to the Executive Officers for resolution pursuant to the preceding sentence (or pursuant to Section 2.1(f)), the Executive Officers shall attempt in good faith to resolve such dispute within [\*\*] days.

(b) If the Executive Officers are unable to resolve a given dispute referred to such Executive Officers pursuant to Section 13.2(a) (or pursuant to Section 2.1(f) (other than Non-Arbitrable Termination Disputes) within [\*\*] days following such referral of such dispute to such Executive Officers, either Party may have the dispute settled by binding arbitration in the manner described below:

(i) Arbitration Request. If a Party intends to begin an arbitration to resolve a dispute arising under a Collaboration Agreement, such Party shall provide written notice (the “Arbitration Request”) to the other Party of such intention and the issues for resolution.

(ii) Additional Issues. Within [\*\*] days after the receipt of the Arbitration Request, the other Party may, by written notice, add additional issues for resolution.

(iii) Arbitration Location; Rules. Except as expressly provided herein, the sole mechanism for resolution of any claim, dispute or controversy arising out of or in connection with or relating to any Collaboration Agreement or the breach or alleged breach thereof shall be binding arbitration by ICC in London, England, pursuant to ICC’s Arbitration Rules and Procedures, except as provided herein.

(iv) English Language. All proceedings shall be held in English and a transcribed record prepared in English. Documents submitted in the arbitration, the originals of which are not in English, shall be submitted together with a reasonably complete and accurate English translation.

(v) Selection of Arbitrators. The Parties shall each select one arbitrator within [\*\*] days after receipt of the Arbitration Request and the two (2) arbitrators so selected shall select by mutual agreement a third arbitrator within [\*\*] days after they have been selected as arbitrators. If all three (3) arbitrators have not been selected within [\*\*] days after receipt of the Arbitration Request or any extension of time that is mutually agreed on, ICC shall select such additional arbitrator(s) needed to complete the three (3) arbitrator panel within [\*\*] days thereafter. If the issues in dispute involve scientific or technical matters, any arbitrators chosen hereunder shall have educational training or experience sufficient to demonstrate a reasonable level of knowledge in the pharmaceutical and biotechnology fields.

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(vi) Time Schedule. Within [\*\*] days after initiation of arbitration, the Parties shall reach agreement upon and thereafter follow procedures directed at assuring that the arbitration will be concluded and the award rendered within no more than [\*\*] months after selection of the three (3) arbitrators. Failing such agreement, ICC will design, and the Parties will follow procedures, directed at meeting such a time schedule.

(vii) Powers of Arbitrators. The arbitrators:

(A) shall not have any power or authority to add to, alter, amend or modify the terms of any Collaboration Agreement;

(B) shall establish and enforce appropriate rules to allow reasonable discovery by the Parties and to ensure that the proceedings, including the decision, be kept confidential and that all Confidential Information of the Parties be kept confidential and be used for no purpose other than the arbitration (unless disclosure or use is otherwise expressly permitted by the applicable Collaboration Agreement);

(C) shall have the power to enforce specifically the applicable Collaboration Agreement and the terms and conditions thereof in addition to any other remedies at law or in equity; and

(D) shall issue all awards in writing.

(viii) Costs; Exclusion from Award. Awards rendered by the arbitrators shall not include costs of arbitration, attorneys’ fees or costs for expert and other witnesses, with respect to which each Party shall bear its own costs and expenses, except that the Parties shall share equally the fees of the arbitrators.

(ix) Injunctive Relief. Nothing in this Agreement (except Section 13.1(a)) shall be deemed as preventing either Party from seeking injunctive relief (or any other provisional remedy such as temporary restraining order, preliminary injunction or other interim equitable relief) from the arbitrators or from any court having jurisdiction over the Parties (and prior to or during any arbitration if necessary to protect the interests of such Party in avoiding irreparable harm or to preserve the status quo pending the arbitration proceeding) and the subject matter of the dispute as necessary to protect either Party’s name, Confidential Information, trade secrets, Know-How or any other proprietary right or otherwise to avoid irreparable harm. In particular, the Parties agree that any breach by a Party of its obligations under Section 6.1, Section 7.5(a) or Article X, or any claim by either Party contrary to Section 13.1(a), will cause irreparable harm to the other Party for which an award of monetary damages would be an inadequate remedy and, accordingly, that the other Party shall be entitled to injunctive relief enjoining such breach without the requirement to post a bond.

(x) Judgment. Judgment on any award rendered by the arbitrators may be entered in any court of competent jurisdiction.

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## ARTICLE XIV INDEMNIFICATION; INSURANCE

Section 14.1 Indemnification by Chiesi. Chiesi shall indemnify, defend and hold harmless uniQure and its Affiliates, and its and their respective directors, officers, employees and agents, from and against any and all liabilities, damages, losses, costs and expenses, including the reasonable fees of attorneys and other professional Third Parties (collectively, “Losses”), arising out of or resulting from any and all Third Party suits, claims, actions, proceedings or demands (“Claims”) to the extent based upon:

(a) any breach of any representation, warranty or covenant made by, or any material obligation of, Chiesi under this Agreement;

(b) Development Program activities conducted by or on behalf (other than by uniQure or any of its Affiliates, or a Third Party performing activities on their behalf) of Chiesi or its Affiliates; or

(c) the gross negligence, recklessness or willful misconduct of Chiesi or its Affiliates and its or their respective directors, officers, employees and agents;

provided that Chiesi shall not be obligated pursuant to this Section 14.1 to the extent uniQure is required to indemnify Chiesi under Section 14.2 below.

Section 14.2 Indemnification by uniQure. uniQure shall indemnify, defend and hold harmless Chiesi and its Affiliates, and its and their respective directors, officers, employees and agents, from and against any and all Losses, arising out of or resulting from any and all Claims to the extent based upon:

(a) any breach of any representation, warranty or covenant made by, or any material obligation of, uniQure under this Agreement;

(b) Development Program activities conducted by or on behalf (other than by Chiesi or any of its Affiliates, or a Third Party performing activities on their behalf) of uniQure or its Affiliates;

(c) the gross negligence, recklessness or willful misconduct of uniQure or its Affiliates and its or their respective directors, officers, employees and agents; or

(d) Claims that the exercise of any rights or licenses granted to Chiesi and its Affiliates in accordance with this Agreement violates or infringes upon the Intellectual Property Rights of any Third Party;

provided that uniQure shall not be obligated pursuant to this Section 14.2 to the extent Chiesi is required to indemnify uniQure under Section 14.1 above.

Section 14.3 Procedure.

(a) A Party entitled to indemnification under this Article XIV (an "Indemnified Party") shall give prompt written notification to the Party from whom

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indemnification is sought (the "Indemnifying Party") of the commencement of any Claim for which indemnification may be sought or, if earlier, upon the assertion of any such Claim by a Third Party (it being understood and agreed, however, that the failure by an Indemnified Party to give notice of a Claim as provided in this Section 14.3(a) shall not relieve the Indemnifying Party of its indemnification obligation under this Agreement except and only to the extent that such Indemnifying Party is actually damaged as a result of such failure to give notice).

(b) Within [\*\*] days after delivery of such notification, the Indemnifying Party may, upon written notice thereof to the Indemnified Party, assume control of the defense of such Claim with counsel reasonably satisfactory to the Indemnified Party.

(c) If the Indemnifying Party does not assume control of such defense, the Indemnified Party shall control such defense and, without limiting the Indemnifying Party's indemnification obligations, the Indemnifying Party shall reimburse the Indemnified Party for all reasonable costs and expenses, including reasonable attorney's fees, incurred by the Indemnified Party in defending itself, within [\*\*] days after receipt of any invoice therefor from the Indemnified Party, such invoice to be issued no more often than quarterly.

(d) The Party not controlling such defense may participate therein at its own expense; provided that, if the Indemnifying Party assumes control of such defense and the Indemnified Party in good faith concludes, based on advice from counsel, that the Indemnifying Party and the Indemnified Party have conflicting interests with respect to such Claim, the Indemnifying Party shall be responsible for the reasonable fees and expenses of counsel to the Indemnified Party in connection with its participation in the defense action.

(e) The Party controlling such defense shall keep the other Party advised of the status of such Claim and the defense thereof and shall consider recommendations made by the other Party with respect thereto.

(f) The Indemnified Party shall not agree to any settlement of any Claim without the prior written consent of the Indemnifying Party, which shall not be unreasonably withheld, delayed or conditioned. The Indemnifying Party shall not, without the prior written consent of the Indemnified Party, agree to any settlement of such Claim, or consent to any judgment in respect thereof, that does not include a complete and unconditional release of the Indemnified Party from all liability with respect thereto, that imposes any liability or obligation on the Indemnified Party or that acknowledges fault by the Indemnified Party.

Section 14.4 Insurance. Each Party shall procure and maintain insurance, including product liability insurance, adequate to cover its obligations hereunder and which are consistent with normal business practices of comparable companies with respect to similar obligations and liabilities, at all times during the Term. uniQure shall further procure and maintain, at uniQure's cost, insurance adequate to cover its obligations under the St. Jude Agreements and Chiesi shall reasonably cooperate with uniQure in obtaining such insurance. It is understood that such insurance shall not be construed to create any limit of either Party's obligations or liabilities with respect to

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its indemnification obligations hereunder. Each Party shall provide the other, upon request, with evidence of such insurance.

Section 14.5 Limitation of Liability. EXCEPT WITH RESPECT TO ANY BREACH BY A PARTY OF ITS OBLIGATIONS UNDER ARTICLE X, EXCEPT FOR ANY DAMAGES ARISING FROM A PARTY'S WILLFUL MISCONDUCT AND EXCEPT TO THE EXTENT A PARTY MAY BE REQUIRED TO INDEMNIFY THE OTHER PARTY UNDER THIS ARTICLE XIV WITH RESPECT TO THIRD PARTY CLAIMS, NEITHER PARTY SHALL BE LIABLE FOR ANY (AND EACH PARTY HEREBY DISCLAIMS ALL) SPECIAL, EXEMPLARY, CONSEQUENTIAL, PUNITIVE OR OTHER INDIRECT DAMAGES, INCLUDING LOST REVENUE AND LOST PROFITS, WHETHER BASED UPON WARRANTY, CONTRACT, TORT, STRICT LIABILITY OR OTHER LEGAL THEORY.

**ARTICLE XV  
MISCELLANEOUS**

Section 15.1 Change of Control. Each Party agrees that, notwithstanding any provisions of this Agreement to the contrary, following the closing of a Change of Control of a Party (the “Acquired Party”), the other Party (the “Non-Acquired Party”) shall not obtain rights or access to the Patent Rights or Know-How of the Acquirer (as defined below) or of the Affiliates of such Acquirer (other than the Acquired Party and its Affiliates which exist immediately prior to the closing of such Change of Control (such Affiliates, the “Pre-Existing Affiliates”)); and the Acquirer and its Affiliates (other than the Acquired Party and its Pre-Existing Affiliates) shall not obtain rights or access to the Patent Rights or Know-How of the Non-Acquired Party or be bound by the restrictions set forth in Section 6.1; provided, however, that the Non-Acquired Party’s rights in all Patent Rights and Know-How of the Acquired Party and its Pre-Existing Affiliates, which Patent Rights and Know-How exist as of the date of the closing of such Change of Control and are then licensed hereunder to the Non-Acquired Party, shall remain licensed to such Non-Acquired Party after the date of the closing of such Change of Control in accordance with and subject to the terms and conditions of this Agreement and shall not be affected in any manner by virtue of such Change of Control. “Acquirer” means, with respect to the Acquired Party, the Third Party that acquires the Control of such Acquired Party.

Section 15.2 Governing Law. The validity and interpretation of this Agreement shall be governed by the laws of England without regard to its conflicts of laws principles and to the express exclusion of the United Nations Conventions on Contracts for the International Sale of Goods (CISG).

Section 15.3 Assignment. Except as expressly provided herein, neither this Agreement nor any rights and obligations hereunder shall be assignable by a Party without the prior written consent of the other Party; provided, however, that a Party may assign this Agreement to any Affiliate or to any successor in interest by way of merger, acquisition or sale of all or substantially all of its assets to which this Agreement relates, provided that such successor agrees in writing to be bound by the terms of this Agreement as if it were the assigning Party. This Agreement shall be binding upon the successors and permitted assigns of the Parties. Any assignment not in accordance with this Section 15.3 shall be void.

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Section 15.4 Entire Agreement; Amendments. This Agreement and the attachments hereto contain the entire understanding and agreement of the Parties with respect to the subject matter hereof and cancel and supersede any and all prior negotiations, correspondence, understandings and agreements between the Parties, whether oral or written, regarding such subject matter, including the Memorandum of Understanding dated 21 December 2012 and the Confidentiality Agreement, but expressly excluding the Commercialization Agreement. Except for the rights expressly conferred on the JSC, JDC or JCC, this Agreement cannot be modified except by a written document bearing the signatures of both Parties. The same applies to any waiver of this written form requirement.

Section 15.5 Notices. Other than as expressly specified in this Agreement, all notices and consents required to be provided hereunder shall be in writing and provided by hand, by recorded delivery mail (return receipt requested), by facsimile, or by recognized overnight courier service to the other Party at its address or facsimile number shown below or such other address or facsimile number notified by such other Party from time to time.

If to uniQure, addressed to:

uniQure Biopharma B.V.  
P.O. Box 22506  
1100 DA Amsterdam  
The Netherlands  
Attention: CEO  
Fax: +31 20 566 9272

If to Chiesi, addressed to:

Chiesi Farmaceutici S.p.A.  
Via Palermo, 26/A  
43122 Parma  
Italy  
Attention: CEO  
Copy to: Corporate Development, Head and General Counsel  
Fax: +39 0521 774468

Section 15.6 Exports. The Parties acknowledge that the export of technical data, materials or products is subject to the exporting Party receiving any necessary export licenses and that the Parties cannot be responsible for any delays attributable to export controls that are beyond the reasonable control of either Party. Chiesi and uniQure agree not to export or re-export, directly or indirectly, any Product (or any associated products, information, items, articles, computer software, media, technical data, the direct product of such data, samples or equipment received or generated under this Agreement) in violation of any Applicable Laws that may be applicable. Chiesi and uniQure agree to obtain similar covenants from their Affiliates and Third Party contractors with respect to the subject matter of this Section 15.6, to the extent applicable.

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Section 15.7 Force Majeure. Neither Party shall lose any rights hereunder or be liable to the other Party for damages or losses on account of failure of performance by the defaulting Party if the failure is occasioned by war, civil insurrection, strike, fire, Act of God, earthquake, tempest, flood, epidemic, blackout, lockout, embargo, governmental acts or orders or restrictions, delays in delivery and non-supply by exclusive suppliers, where such delay or non-supply occurs as a result of such force majeure, or any other reason where failure to perform is beyond the reasonable control of such Party and such failure to perform is not caused by the negligence, intentional conduct or misconduct of the non-performing Party and such Party has exerted all Commercially Reasonable Efforts to avoid or remedy such force majeure event; provided, however, that in no event shall a Party be required to settle any labor dispute or disturbance.

Section 15.8 Performance by Affiliates, Sub-distributors or Third Party Contractors. To the extent that this Agreement imposes obligations on or permits the exercise of rights by Affiliates, Sub-distributors or Third Party contractors of a Party, such Party shall cause such Party’s Affiliates, Sub-distributors or Third Party contractors to perform such obligations (and all related obligations) and shall remain responsible for any breach of such obligations and for the exercise of rights by such Party’s Affiliates, Sub-distributors or Third Party Contractors.

Section 15.9 Independent Contractors. It is understood and agreed that the relationship between the Parties hereunder is that of independent contractors and that nothing in this Agreement shall be construed as authorization for either uniQure or Chiesi to act for, bind or commit the other in any way. Each Alliance Manager shall be considered the employee of Chiesi or uniQure, as the case may be, and shall not be deemed to be an employee of the other Party.

Section 15.10 Costs. Except as expressly provided in this Agreement or as separately agreed upon in writing between the Parties, each Party shall bear its own costs incurred in connection with the implementation of the obligations under this Agreement.

Section 15.11 Construction. Each Party agrees that this Agreement shall be interpreted without regard to any presumption or rule requiring construction against the Party causing this Agreement to be drafted.

Section 15.12 English Language. This Agreement was prepared and is established in the English language; any translation thereof shall be deemed for convenience only and shall never prevail against the original English version. All reports, notices and communications to be exchanged under this Agreement shall be in the English language; provided, however, that, neither Party shall be under any obligation to translate into English any document originally established and existing in another language, for the sole purpose of communicating such document to the other Party, it being agreed that such documents will be provided on an as-is basis.

Section 15.13 No Implied Waivers; Rights Cumulative. No failure on the part of a Party to exercise, and no delay in exercising, any right, power, remedy or privilege under this Agreement, or provided by statute or at law or in equity or otherwise, shall impair, prejudice or constitute a waiver of any such right, power, remedy or privilege or be construed as a waiver of any breach of this Agreement or as

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an acquiescence therein, nor shall any single or partial waiver of any such right, power, remedy or privilege preclude any other or further exercise thereof or the exercise of any other right, power, remedy or privilege. Any Party may waive its rights hereunder in a writing signed by such Party.

Section 15.14 Severability. If, under Applicable Law, any provision of this Agreement is held to be invalid or unenforceable, or otherwise directly or indirectly affects the validity of any other material provision(s) of this Agreement (such invalid or unenforceable provision, a “Severed Clause”), this Agreement shall endure except for the Severed Clause. The Parties shall consult one another and use reasonable efforts to agree upon a valid and enforceable provision that is a reasonable substitute for the Severed Clause in view of the objectives contemplated by the Parties when entering into the Agreement and the general balance of the respective interests of the Parties as initially intended under the Agreement.

Section 15.15 Counterparts. This Agreement may be executed in two (2) or more counterparts, each of which will be deemed an original, but all of which together will constitute one and the same instrument. A pdf file of this Agreement contained in an email, including the signed signature pages hereto, will be deemed to be an original.

[Signature Page Follows]

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IN WITNESS WHEREOF, the Parties have executed this Co-Development and License Agreement as of the Effective Date.

**UNIQURE BIOPHARMA B.V.**

By: /s/ Piers Morgan  
Name: Mr. Piers Morgan  
Title: Chief Financial Officer

**UNIQURE BIOPHARMA B.V.**

By: /s/ Hans Preusting  
Name: Mr. Hans Preusting  
Title: Business Development, Vice President

**CHIESI FARMACEUTICI S.p.A.**

By: /s/ Alberto Chiesi  
Name: Mr. Alberto Chiesi  
Title: President

**CHIESI FARMACEUTICI S.p.A.**

By: /s/ Ugo Di Francsco  
Name: Mr. Ugo Di Francesco  
Title: CEO

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## EXHIBIT A

### Initial Development Plan and Budget

#### AMT-060 Key Development Activities to MAA

Foregrounds and assumptions to the Development Plan

- The present document is a high level overview and not an exhaustive list of all the work and activities involved in the development of AMT060.
- The timelines have been developed based on current knowledge and understanding of what is necessary in order to obtain marketing authorization.

- Plans and decisions may change depending on emerging data and information, according to the decisions taken by the JDC.
- The present plan does not include some tasks/ activities that are already foreseen, but which cannot be planned at the present stage due to missing information or for which decision making is expected to occur later on in development. Those activities include:

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Tables 1 and 2 below highlight **key activities** in the AMT-060 development programme.

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Table 1: Key activities needed to deliver Phase I

uniQure Function	Activity	Start Date (1)	Completion Date (2)	Status
Regulatory affairs Non-clinical	[**]	[**]	[**]	[**]
	[**]	[**]	[**]	[**]
	[**]	[**]	[**]	[**]
Process Development	[**]	[**]	[**]	[**]
	[**]	[**]	[**]	[**]
Assay Development	[**]	[**]	[**]	[**]
	[**]	[**]	[**]	[**]
	[**]	[**]	[**]	[**]
Quality control	[**]	[**]	[**]	[**]
	[**]	[**]	[**]	[**]
Manufacturing	[**]	[**]	[**]	[**]
Quality Assurance	[**]	[**]	[**]	[**]
Clinical	[**]	[**]	[**]	[**]
	[**]	[**]	[**]	[**]

(1) “Start date” means the date when the activity is planned to start. For clinical studies, “start date” means the first patient enrolled in the study (i.e. [\*\*]). For regulatory activities, it means the start of the window period in which the activity will occur.

(2) “Completion date” means the date by which the activity is planned to end. For clinical studies, “completion date” means the completion of the Clinical Study Report (i.e. CSR). For regulatory activities, it means the end of the window period in which the activity will occur.

(3) The planned “completion date” for the Phase I study assumes:[\*\*].

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Table 2: Key activities needed to deliver Phase II/III

uniQure Function	Activity	Start Date (1)	Completion Date (2)	Status
Regulatory affairs	[**]	[**]	[**]	[**]
	[**]	[**]	[**]	[**]
	[**]	[**]	[**]	[**]
Non clinical	[**]	[**]	[**]	[**]
Process Development	[**]	[**]	[**]	[**]
	[**]	[**]	[**]	[**]
	[**]	[**]	[**]	[**]
Assay Development	[**]	[**]	[**]	[**]
	[**]	[**]	[**]	[**]
	[**]	[**]	[**]	[**]
Quality control	[**]	[**]	[**]	[**]
Manufacturing	[**]	[**]	[**]	[**]
Quality Assurance	[**]	[**]	[**]	[**]
Clinical	[**]	[**]	[**]	[**]
	[**]	[**]	[**]	[**]

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FTE requirements over the next [\*\*]

Table 3 shows the maximum FTE required by UniQure per year over the next [\*\*]. This timeline captures [\*\*].

- These numbers include [\*\*].
- The figures also include [\*\*].

Table 4 indicates the maximum number of FTEs requirements for the time period [\*\*] and Table 5 shows [\*\*].

NOTE: FTEs and costs beyond [\*\*] to be determined and agreed by the JDC.



Table 3: Maximum FTE number per year per Function

uniQure Function*			FTE AV.[**]	FTE AV.[**]
[**]			[**]	[**]
[**]			[**]	[**]
[**]	[**]		[**]	[**]
	[**]	[**]	[**]	[**]
[**]		[**]	[**]	[**]
		[**]	[**]	[**]
	[**]		[**]	[**]

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[**]		[**]	[**]
[**]		[**]	[**]
[**]	[**]	[**]	[**]
	[**]	[**]	[**]
[**]		[**]	[**]
[**]		[**]	[**]
AV. TOTAL		[**]	[**]

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Table 4: Maximum FTE requirement [\*\*]:

Year	[**]	[**]	[**]
Total FTE	[**]	[**]	[**]
[**]	[**]	[**]	[**]
[**]	[**]	[**]	[**]
[**]	[**]	[**]	[**]
[**]	[**]	[**]	[**]
[**]	[**]	[**]	[**]
[**]	[**]	[**]	[**]
[**]	[**]	[**]	[**]
[**]	[**]	[**]	[**]

Table 5: AMT-060 total budget and costs [\*\*]

	[**]	[**]	[**]	[**]	[**]	[**]
[**]	[**]	[**]	[**]	[**]	[**]	[**]
[**]	[**]	[**]	[**]	[**]	[**]	[**]
[**]	[**]	[**]	[**]	[**]	[**]	[**]
[**]						[**]
[**]	[**]	[**]	[**]	[**]	[**]	[**]
TOTAL BUDGET (€)	[**]	[**]	[**]	[**]	[**]	[**]

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NOTE: all external costs estimates and costs allocation per year are based on current knowledge and planning.

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Outline of proposed clinical studies

Phase I study

Study

Phase I, multicentre, open label, prospective, interventional, single dose, dose-escalation clinical trial to investigate the safety and tolerability of AAV5-hFIXco, an adeno-associated viral vector containing a codon-optimised human factor IX gene in severe Haemophilia B (Study code: AMT-060-001)

Objectives and end-points

Primary objective:

To assess the safety of systemic and determine the MTD of the gene therapy vector AAV5-hFIXco for the treatment of the Severe Haemophilia B, registering and evaluating the occurrence of adverse events and serious adverse events at the dose identified.

Main secondary objectives:

- To estimate the appropriate dose required to achieve stable expression of hFIX at or above 3% of normal
- To evaluate kinetics (dose-related duration and magnitude) of expression
- To describe the immune response to hFIX transgene product
- To describe the immune response to the AAV5 capsid proteins
- To assess viral shedding in various body fluids (including semen)
- Assess the occurrence of FIX inhibitors
- Evaluate coagulation parameters
- Assess need for FIX concomitant treatment

**Study design** [\*\*]

**Study population (key eligibility criteria)** [\*\*]

**Treatments** [\*\*]

**Duration** The trial will last [\*\*]for each patient

**Sample size** [\*\*]patients

**Phase II/ III:** [\*\*].

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## EXHIBIT B

### Certain Requirements under PHS Agreements

*Each capitalized term used but not defined in this Exhibit B shall have the meaning ascribed to it in the applicable PHS Agreement.*

#### PHS 2011 Requirements

- 4.2 Licensee agrees that any sublicenses granted by it shall provide that the obligations to PHS of Paragraphs 5.1-5.4, 8.1, 10.1, 10.2, 12.5, and 13.8-13.10 of this Agreement shall be binding upon the sub-licensee as if it were a party to this Agreement. Licensee further agrees to attach copies of these Paragraphs to all sublicense agreements.
- 5.1 (a) PHS reserves on behalf of the Government an irrevocable, nonexclusive, nontransferable, royalty-free license for the practice of all inventions licensed under the Licensed Patent Rights throughout the world by or on behalf of the Government and on behalf of any foreign government or international organization pursuant to any existing or future treaty or agreement to which the Government is a signatory. Prior to the First Commercial Sale, Licensee agrees to provide PHS with reasonable quantities of Licensed Products or materials made through the Licensed Processes for PHS research use; and
- (b) In the event that the Licensed Patent Rights are Subject Inventions made under a Cooperative Research and Development Agreement ("CRADA"), Licensee grants to the Government, pursuant to 15 U.S.C. §3710a(b)(1)(A), a nonexclusive, nontransferable, irrevocable, paid-up license to practice Licensed Patent Rights or have Licensed Patent Rights practiced throughout the world by or on behalf of the Government. In the exercise of this license, the Government shall not publicly disclose trade secrets or commercial or financial information that is privileged or confidential within the meaning of 5 U.S.C. §552(b)(4) or which would be considered as such if it had been obtained from a non-Federal party. Prior to the First Commercial Sale, Licensee agrees to provide PHS reasonable quantities of Licensed Products or materials made through the Licensed Processes for PHS research use.
- 5.2 Licensee agrees that products used or sold in the United States embodying Licensed Products or produced through use of Licensed Processes shall be manufactured substantially in the United States, unless a written waiver is obtained in advance from PHS.
- 5.3 Licensee acknowledges that PHS may enter into future CRADAs under the Federal Technology Transfer Act of 1986 that relate to the subject matter of this Agreement. Licensee agrees not to unreasonably deny requests for a Research License from future collaborators with PHS when acquiring these rights is necessary in order to make a CRADA project feasible. Licensee may request an opportunity to join as a party to the proposed CRADA.
- 5.4 (a) In addition to the reserved license of Paragraph 5.1, PHS reserves the right to grant Research Licenses directly or to require Licensee to grant Research Licenses on reasonable terms. The purpose of these Research Licenses is to encourage basic

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research, whether conducted at an academic or corporate facility. In order to safeguard the Licensed Patent Rights, however, PHS shall consult with Licensee before granting to commercial entities a Research License or providing to them research samples of materials made through the Licensed Processes; and

(b) In exceptional circumstances, and in the event that Licensed Patent Rights are Subject Inventions made under a CRADA, the Government, pursuant to 15 U.S.C. §3710a(b)(1)(B), retains the right to require the Licensee to grant to a responsible applicant a nonexclusive, partially exclusive, or exclusive sublicense to use the Licensed Patent Rights in the Licensed Field of Use on terms that are reasonable under the circumstances, or if Licensee fails to grant this license, the Government retains the right to grant the license itself. The exercise of these rights by the Government shall only be in exceptional circumstances and only if the Government determines:

- (i) the action is necessary to meet health or safety needs that are not reasonably satisfied by Licensee;

(ii) the action is necessary to meet requirements for public use specified by Federal regulations, and these requirements are not reasonably satisfied by the Licensee; or

(iii) the Licensee has failed to comply with an agreement containing provisions described in 15 U.S.C. §3710a(c)(4)(B); and

(c) The determination made by the Government under this Paragraph 5.4 is subject to administrative appeal and judicial review under 35 U.S.C. §203(b).

- 10.1 Licensee shall use its reasonable commercial efforts to bring the Licensed Products and Licensed Processes to Practical Application. “Reasonable commercial efforts” for the purposes of this provision shall include adherence to the Commercial Development Plan in Appendix E and performance of the Benchmarks in Appendix D. The efforts of a sub-licensee shall be considered the efforts of Licensee.
- 10.2 Upon the First Commercial Sale, until the expiration or termination of this Agreement, Licensee shall use its reasonable commercial efforts to make Licensed Products and Licensed Processes reasonably accessible to the United States public.
- 12.5 Licensee shall indemnify and hold PHS, its employees, students, fellows, agents, and consultants harmless from and against all liability, demands, damages, expenses, and losses, including but not limited to death, personal injury, illness, or property damage in connection with or arising out of:
- (a) the use by or on behalf of Licensee, its sub-licensees, directors, employees, or third parties of any Licensed Patent Rights; or
- (b) the design, manufacture, distribution, or use of any Licensed Products, Licensed Processes or materials by Licensee, or other products or processes developed in connection with or arising out of the Licensed Patent Rights.
- 13.8 PHS reserves the right according to 35 U.S.C. §209(d)(3) to terminate or modify this Agreement if it is determined that this action is necessary to meet the requirements for

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public use specified by federal regulations issued after the date of the license and these requirements are not reasonably satisfied by Licensee.

- 13.9 Within [\*\*] days of receipt of written notice of PHS’ unilateral decision to modify or terminate this Agreement, Licensee may, consistent with the provisions of 37 C.F.R. §404.11, appeal the decision by written submission to the designated PHS official. The decision of the designated PHS official shall be the final agency decision. Licensee may thereafter exercise any and all administrative or judicial remedies that may be available.
- 13.10 Within [\*\*] days of expiration or termination of this Agreement under this Article 13, a final report shall be submitted by Licensee. Any royalty payments, including those incurred but not yet paid (such as the full minimum annual royalty), and those related to patent expense, due to PHS shall become immediately due and payable upon termination or expiration. If terminated under this Article 13, sub-licensees may elect to convert their sublicenses to direct licenses with PHS pursuant to Paragraph 4.3. Unless otherwise specifically provided for under this Agreement, upon termination or expiration of this Agreement, Licensee shall return all Licensed Products or other materials included within the Licensed Patent Rights to PHS or provide PHS with certification of the destruction thereof. Licensee may not be granted additional PHS licenses if the final reporting requirement is not fulfilled.

#### PHS 2007 Requirements

- 4.2 Licensee agrees that any sublicenses granted by it shall provide that the obligations to PHS of Paragraphs 5.1, 5.2, 8.1, 10.1, 10.2, 12.5 and 13.6-13.8 of this Agreement shall be binding upon the sub-licensee as if it were a party to this Agreement. Licensee further agrees to attach copies of these Paragraphs to all sublicense Agreements.
- 5.1 Prior to the First Commercial Sale, Licensee agrees to provide PHS with reasonable quantities of Licensed Products or New Products made through the Licensed Processes or Supplied Materials solely for PHS research use, if requested in writing.
- 5.2 Licensee agrees that products used or sold in the United States embodying Licensed Products or New Products or produced through use of Licensed Processes shall be manufactured substantially in the United States, unless a written waiver is obtained in advance from PHS.
- 8.1 Licensee agrees to keep accurate and correct records of Licensed Products or New Products made, used, sold, or imported and Licensed Processes practiced under this Agreement appropriate to determine the amount of royalties due PHS. These records shall be retained for at least [\*\*] years following a given reporting period and shall be available during normal business hours for inspection, at the expense of PHS, by an accountant or other designated auditor selected by PHS for the sole purpose of verifying reports and royalty payments made under this Agreement. The accountant or auditor shall only disclose to PHS information relating to the accuracy of reports and royalty payments made under this Agreement. If an inspection shows an underreporting or underpayment in excess of [\*\*] percent ([\*\*]%) for any twelve (12) month period, then Licensee shall reimburse PHS for the cost of the inspection at the time Licensee pays the unreported royalties, including any additional royalties as required by

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Paragraph 9.8, All royalty payments required under this Paragraph shall be due within [\*\*] days of the date PHS provides Licensee notice of the payment due.

- 10.1 Licensee shall use its reasonable commercial efforts to bring the Licensed Products or New Products and Licensed Processes to Practical Application. “Reasonable commercial efforts” for the purposes of this provision shall include adherence to the Commercial Development Plan in Appendix E and performance of the Benchmarks in Appendix D. The efforts of a sublicense shall be considered the efforts of Licensee.
- 10.2 Upon the First Commercial Sale, until the expiration or termination of this Agreement, Licensee shall use its reasonable commercial efforts to make Licensed Products or New Products and Licensed Processes reasonably accessible to the United States public.

- 12.5 Licensee shall indemnify and hold PHS, its employees, students, fellows, agents, and consultants harmless from and against all liability, demands, damages, expenses, and losses, including but not limited to death, personal injury, illness, or property damage in connection with or arising out of:
- (a) the use by or on behalf of Licensee, its sub-licensees, its directors, employees, or third parties of any Licensed Patent Rights or Supplied Materials; or
  - (b) the design, manufacture, distribution, or use of any Licensed Products, Licensed Processes or New Products by Licensee, or other products or processes developed in connection with or arising out of the Licensed Patent Rights. Licensee agrees to maintain a liability insurance program consistent with sound business practice.
- 13.6 In making the determination referenced in Paragraph 13.5, PHS shall take into account the normal course of such commercial development programs conducted with sound and reasonable business practices and judgment and the annual reports submitted by Licensee under Paragraph 9.2. Prior to invoking termination or modification of this Agreement under Paragraph 13.5, PHS shall give written notice to Licensee providing Licensee specific notice of, and a [\*\*] day opportunity to respond to. PHS' concerns as to the items referenced in 13.5(a)-13.5(g). If Licensee fails to alleviate PHS' concerns as to the items referenced in 13.5(a)-13.5(g) or fails to initiate corrective action to PHS' satisfaction, PHS may terminate this Agreement.
- 13.7 PHS reserves the right according to 35 U.S.C. §209(d)(3) to terminate or modify this Agreement if it is determined that the action is necessary to meet the requirements for public use specified by federal regulations issued after the date of the license and these requirements are not reasonably satisfied by Licensee.
- 13.8 Within [\*\*] days of receipt of written notice of PHS' unilateral decision to modify or terminate this Agreement, Licensee may, consistent with the provisions of 37 CFR §404.11, appeal the decision by written submission to the designated PHS official. The decision of the designated PHS official shall be the final agency decision. Licensee may thereafter exercise any and all administrative or judicial remedies that maybe available.

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## EXHIBIT C

### Approved Activities

1. Estimated Product expenses for [\*\*]:

Approved Activity	Subtotal		
	[**]	[**]	[**]
[**]	[**]	[**]	[**]
[**]	[**]	[**]	[**]
[**]	[**]	[**]	[**]

**TOTAL** Product Development Costs (out-of-pocket) for [\*\*]

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## EXHIBIT D

### Certain Terms for HemB Supply and Distribution Agreement

**Transfer Price:** Chiesi shall purchase the Product for the greater of (a) [\*\*] or (b) [\*\*] (the “Product Transfer Price”). The Product Manufacturing Cost Reimbursement associated with each patient dose of the Product sold by Chiesi shall be credited against the Product Transfer Price. The “Product Manufacturing Cost Reimbursement” for each patient dose of the Product shall be uniQure’s Fully Loaded Costs of Goods.

**Commercial Supply:** Prior to initiation of the Pivotal Study, the Parties shall negotiate in good faith the HemB Supply and Distribution Agreement for commercial material with the financial terms described above and other terms expected to be substantially similar to the Commercialization Agreement. For the avoidance of doubt, the provisions set forth in Section 2.6 of the Commercialization Agreement shall be included in the HemB Supply and Distribution Agreement.

“Fully Loaded Costs of Goods” shall be determined according to the principles applied in the Commercialization Agreement, in particular Schedule 2.3 thereof.

#### Indemnification:

Indemnification by Chiesi. Chiesi shall indemnify, defend and hold harmless uniQure and its Affiliates, and its and their respective directors, officers, employees and agents, from and against any and all Losses, arising out of or resulting from any and all Third Party Claims to the extent based upon:

(a) any breach of any representation, warranty or covenant made by, or any material obligation of, Chiesi under the HemB Supply and Distribution Agreement;

(b) the gross negligence, recklessness or willful misconduct of Chiesi or its Affiliates and its or their respective directors, officers, employees and agents; or

(c) any theory of product liability (including without limitation tort, warranty, or strict liability) that is applicable in the Territory with respect to the death, personal injury, or illness of any Person in the Territory, and arising directly from Chiesi’s or its Affiliates’ or Sub-distributors’ Commercialization of the Product in the Territory;

provided that Chiesi shall not be obligated to indemnify uniQure to the extent uniQure is required to indemnify Chiesi under the HemB Supply and Distribution Agreement.

Indemnification by uniQure. uniQure shall indemnify, defend and hold harmless Chiesi and its Affiliates, and its and their respective directors, officers, employees and agents, from and against any and all Losses, arising out of or resulting from any and all Claims to the extent based upon:

(a) any breach of any representation, warranty or covenant made by, or any material obligation of, uniQure under the HemB Supply and Distribution Agreement;

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(b) the gross negligence, recklessness or willful misconduct of uniQure or its Affiliates and its or their respective directors, officers, employees and agents;

(c) any theory of product liability (including without limitation tort, warranty, or strict liability) that is applicable in the Territory with respect to the death, personal injury, or illness of any Person in the Territory, and arising directly from uniQure’s or its Affiliates’ design, Manufacture, storage, release and handling of the Product; or

(d) Claims that the (i) Commercialization of the Product; or (ii) exercise of any rights or licenses granted to Chiesi and its Affiliates in accordance with the HemB Supply and Distribution Agreement; violates or infringes upon the Intellectual Property Rights of any Third Party;

provided that uniQure shall not be obligated to indemnify Chiesi to the extent Chiesi is required to indemnify uniQure under the HemB Supply and Distribution Agreement.

**Trademark:** The Product will be Commercialized by Chiesi in the Field in the Territory exclusively under a trademark mutually agreed between the Parties. Such trademark shall be owned by uniQure and shall be licensed to Chiesi for Commercialization of the Product in the Field in the Territory. Section 2.2(a) of the Commercialization Agreement shall apply *mutatis mutandis*.

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EXHIBIT E

Licensed Patents

***								
**	**	**	**	**	**	**		
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Note:

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**EXHIBIT F****uniQure FTE Limits**

Unless otherwise agreed in writing as part of the then current Development Plan and Budget, the number of uniQure FTEs will be no higher than:

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Unless otherwise agreed in writing as part of the then current Development Plan and Budget, the number of Chiesi FTEs will be no higher than:

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**EXHIBIT G****Technology Transfer**

The following is a non-exhaustive list describing key steps which the Parties would typically envisage for a transfer of the Manufacturing of the Product to another manufacturing site during Product Development:

Steps	Estimated Timelines
· if transferred to a Third Party manufacturer: select and contract manufacturer party	[**]
· process transfer (on paper)	[**]
· obtain time slot	[**]
· process validation	[**]
<b>Total</b>	<b>[**]</b>

G-1

**EXHIBIT H****Disclosure Schedule to Section 11.2(e)**

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G-1

Confidential Materials omitted and filed separately with the Securities and Exchange Commission. Double asterisks denote omissions.

## COMMERCIALIZATION AGREEMENT

This Commercialization Agreement (this “Agreement”) is entered into as of 29 April 2013 (the “Effective Date”), by and between uniQure Biopharma B.V., formerly known as Amsterdam Molecular Therapeutics (AMT) B.V., a Dutch corporation, with its offices at Meibergdreef 61, 1105 BA Amsterdam, The Netherlands (“uniQure”), and Chiesi Farmaceutici S.p.A., an Italian corporation, with its offices at Via Palermo, 26/A, 43122 Parma, Italy (“Chiesi”). uniQure and Chiesi are sometimes referred to herein individually as a “Party” and collectively as the “Parties”.

WHEREAS, uniQure is a company engaged in the research and clinical development of human gene based therapies. Its lead product, “Glybera”, for the treatment of lipoprotein lipase deficiency was approved by the European Commission in November 2012;

WHEREAS, Chiesi is a pharmaceutical company engaged in the research, development, sales and marketing as well as distribution of ethical medicinal products;

WHEREAS, uniQure desires to appoint Chiesi, on an exclusive basis, to obtain and maintain the best possible Price and Reimbursement Approval (as defined below) and to Commercialize (as defined below) the Product (as defined below) in the Territory (as defined below), in accordance with the terms and conditions set forth below, and Chiesi desires to accept uniQure’s exclusive appointment.

NOW, THEREFORE, uniQure and Chiesi hereby agree as follows:

### ARTICLE I DEFINITIONS; INTERPRETATION

Capitalized terms used herein shall have the meanings assigned to them as follows.

- 1.1 “Affiliate” shall mean, with respect to a Party, any Person Controlled by, in Control of, or under common Control with such Party.
- 1.2 “Additional Rights” has the meaning set forth in Section 7.5(a).
- 1.3 “Agreement” has the meaning set forth in the first and opening paragraph of this Agreement.
- 1.4 “Alliance Manager” has the meaning set forth in Section 4.4.
- 1.5 “Applicable Laws” shall mean all applicable laws, statutes, codes, rules and regulations, judgments, order and ordinances, including any rules, regulations, guidelines or other requirements of any Regulatory Authority within the Territory, that may be in effect from time to time.
- 1.6 “Approved Activities” has the meaning set forth in Section 8.1(b).

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### CONFIDENTIAL

1.7 “Average Net Sales Price” shall mean the average net sales price of a particular Product in the Territory, calculated on a monthly basis, by dividing the Net Sales of the Product in the Territory effected in a particular calendar month by the number of patient doses of the Product accounting for the Net Sales in such calendar month.

1.8 “Business Day” shall mean a day on which banking institutions in Amsterdam, The Netherlands and Parma, Italy, are open for business, excluding any Saturday or Sunday.

1.9 “Certificate of Analysis” shall mean the certificate substantially in the form attached hereto as Schedule 1.9 evidencing the analytical test conducted on a specific lot of Product and setting forth, among other items, the items tested, Specifications, and test results.

1.10 “Certificate of Compliance” shall mean the certificate substantially in the form attached as Schedule 1.10 stating that a specific lot of Product complies with the warranties set forth in Section 5.2.

1.11 “Chiesi” has the meaning set forth in the first and opening paragraph of this Agreement.

1.12 “Claims” has the meaning set forth in Section 6.1.

1.13 “Co-Development and License Agreement” shall mean that certain Co-Development and License Agreement for Hemophilia B concluded separately between the Parties on the date hereof.

1.14 “Collaboration” shall mean the relationship between and activities conducted by the Parties under this Agreement and all other agreements between the Parties referenced herein (other than the Confidentiality Agreement), including the Co-Development and License Agreement (collectively, the “Collaboration Agreements”).

1.15 “Collaboration Agreements” has the meaning set forth in Section 1.14.

1.16 “Commercialization” shall mean any and all activities, whether before or after Regulatory Approval, directed to the marketing, detailing and promotion of the Product and shall include pre-launch, launch and post-launch marketing, promoting, detailing, marketing research, medical affairs, managed markets, distributing, offering to commercially sell and commercially selling the Product, importing, exporting or transporting the Product for commercial sale and

regulatory affairs with respect to the foregoing, including the filing and obtaining of Price and Reimbursement Approval for the Product, but shall not include Manufacturing nor any development activities. When used as a verb, “Commercializing”, “Commercialize” and “Commercialized” shall mean to engage in Commercialization.

1.17 “Commercially Reasonable Efforts” shall mean, with respect to the efforts to be expended by a Party with respect to a goal, reasonable, diligent, good faith efforts to accomplish such goal as a similarly situated (with respect to size and assets) pharmaceutical company would use to accomplish a similar goal under similar circumstances so as to achieve such goal as expeditiously as possible; provided that, with

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respect to the Commercialization of the Product, such efforts shall be substantially equivalent to those efforts and resources that a similarly situated (with respect to size and assets) pharmaceutical company would typically devote to its own internally discovered products of similar market potential at a similar stage in their product life so as to achieve such goal as expeditiously as possible (which, with respect to activities for which Chiesi is responsible, shall be without regard to any amounts paid or payable to uniQure with respect to the Product under this Agreement or the Co-Development and License Agreement). Without prejudice to the foregoing and with respect to Chiesi, Commercially Reasonable Efforts shall at least include the efforts as further described in Schedule 8.1(a).

1.18 “Confidential Information” shall mean all confidential or proprietary information of a Party, including information regarding such Party’s or its Affiliates’ products, business, business plans, financial status, biological substances, chemical substances, formulations, techniques, methodology, equipment, sources of supply and patent positioning and information belonging to such Party’s Affiliate or a Third Party and provided to the other Party under this Agreement. The terms and conditions of this Agreement shall be deemed “Confidential Information” of both Parties. All information disclosed by uniQure prior to the Effective Date pursuant to the Two Way Confidentiality Disclosure Agreement between Amsterdam Molecular Therapeutics (AMT) B.V. and Chiesi Farmaceutici S.p.A. dated 22 July 2010 (the “Confidentiality Agreement”) shall be deemed “Confidential Information” of uniQure hereunder.

1.19 “Confidentiality Agreement” has the meaning set forth in Section 1.18.

1.20 “Confirmed Firm Order” has the meaning set forth in Section 2.4(c).

1.21 “Control” or “Controlled” shall mean, (a) when used in reference to any Confidential Information, Patent or other Intellectual Property Rights, the possession (whether by ownership or license (other than solely pursuant to a license under this Agreement)) by such Party or any of its Affiliates, of the legal authority or right to grant to the other Party access or a license or sublicense to such Confidential Information, Patent or other Intellectual Property Rights as provided herein, without violating the terms of any agreement or arrangement with any Third Party, or (b) when used in reference to Section 1.1, (i) the possession, directly or indirectly, of the power to direct the management or policies of a Person, whether through ownership of voting securities, by contract or otherwise; (ii) ownership of fifty percent (50%) or more of the voting securities entitled to vote for the election of directors in the case of a corporation, or fifty percent (50%) or more of the equity interest in the case of any other type of legal entity; or (iii) status as a general partner in any partnership, or any other arrangement whereby a Person controls or have the right to control the board of directors or equivalent governing body of a corporation or other Person. Notwithstanding the foregoing, any portfolio company of any stockholder of such Person (which stockholder is a venture capital fund or private equity fund) shall not be deemed to be “under common Control with” such Person.

1.22 “Controlling Party” has the meaning set forth in Section 7.5(b).

1.23 “Cover” or “Covered” shall mean, with respect to any Patent and the subject matter at issue, that, but for a license granted under a Valid Claim of such Patent, the manufacture, use, sale, offer for sale or importation of the subject matter at issue

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would infringe such Valid Claim, or, in the case of a Patent that is a patent application, would infringe a Valid Claim in such patent application if it were to issue as a patent.

1.24 “Delivery Notification” has the meaning set forth in Section 2.5(b).

1.25 “Discretionary Manufacturing Changes” has the meaning set forth in Section 3.4(b).

1.26 “Effective Date” has the meaning set forth in the first and opening paragraph of this Agreement.

1.27 “EMA” shall mean the European Medicines Agency and any successor agency thereto.

1.28 “EU” shall mean the European Union.

1.29 “EU Member States” shall mean Austria, Belgium, Bulgaria, Cyprus, the Czech Republic, Denmark, Estonia, Finland, France, Germany, Greece, Hungary, Ireland, Italy, Latvia, Lithuania, Luxembourg, Malta, the Netherlands, Poland, Portugal, Romania, Slovakia, Slovenia, Spain, Sweden and the United Kingdom.

1.30 “Executive Officers” shall mean the Chief Executive Officer of Chiesi or a senior officer designated by Chiesi, and the Chief Executive Officer of uniQure or a senior officer designated by uniQure.

1.31 “Existing Third Party Licenses” has the meaning set forth in Section 7.4.

1.32 “EXW” shall mean “ex works” as defined by the International Chamber of Commerce (Incoterms 2010).

1.33 “Failure to Supply” has the meaning set forth in Section 2.6(a).

1.34 “FDA” shall mean the US Food and Drug Administration and any successor agency thereto.

1.35 “Field” shall mean the treatment of lipoprotein lipase deficiency.



1.36 “Firm Order” shall mean a written (including facsimile or email) irrevocable firm purchase order for the Product, which order shall include the precise name of the Product ordered and the quantity of the Product ordered (such quantity to be equal or above the Minimum Order Quantity).

1.37 “First Commercial Sale” shall mean the first sale by Chiesi, an Affiliate of Chiesi, or a Sub-distributor of Chiesi, as the case may be, of the Product to a Third Party in the Territory; provided, however, that neither (a) transfers of the Product between Chiesi and its Affiliates or Sub-distributors nor (b) supply of the Product for clinical trial purposes, shall constitute a First Commercial Sale.

1.38 “Force Majeure Event” has the meaning set forth in Section 11.6.

1.39 “Forecast” has the meaning set forth in Section 2.4(a).

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1.40 “FTE” shall mean an annual average of at least [\*\*] hours allocated to one or more persons allocated to the Commercialization of the Product (including product specialists, KAMs, MSLs, medical, regulatory, pharmacovigilance, market access and marketing personnel) in the Territory (both at an headquarter and country level).

1.41 “Fully Loaded Cost of Goods” shall mean the fully loaded cost of goods of the Product as defined in Schedule 2.3.

1.42 “GDPs” shall mean the current good distribution practices promulgated by any Regulatory Authorities or Applicable Laws throughout the Territory that are applicable to the Product.

1.43 “Gene Therapy” shall mean the introduction and expression of genetic material in cells of a person in order to cure a disease or to minimize disease symptoms.

1.44 “Glybera Manufacturing Cost Reimbursement” has the meaning set forth in Section 2.3(c)(i).

1.45 “GMPs” shall mean the current good manufacturing practices promulgated by any Regulatory Authorities or Applicable Laws throughout the Territory that are applicable to the Product.

1.46 “Improvements” shall mean any improvements to the Product Controlled by uniQure during the Term, such as future formulations, dosages, dosage forms, delivery modes and line extensions of the Product, packaging of the Product, labeling of the Product, and developments in the Product itself.

1.47 “Indemnified Party” has the meaning set forth in Section 6.3(i).

1.48 “Indemnifying Party” has the meaning set forth in Section 6.3(i).

1.49 “Intellectual Property Rights” shall mean all patents (including the Patents), trademarks (including the Trademark), trade names, service marks, trade dress, trade secrets and copyrights, including, without limitation, any renewal, extension or other rights therefor, and applications, provisionals, divisionals, reexaminations, continuations in part, divisions, continuations, reissues, additions, substitutions and registrations for any of the foregoing and all corresponding foreign patents and patent applications of each of the foregoing, technical information, devices, processes, procedures, discoveries, techniques, formulae, software, designs, drawings, data, methods, protocols, products, apparatuses and other materials, compositions, mask works, domain names, schematics, manufacturing processes, know-how, moral rights, software programs or applications, manufacturing and production processes and techniques, research and development information, drawings, specifications, designs, plans, proposals, technical data, results of experimentation and testing (whether or not patentable) in written, electronic, physical (including in the form of tangible compounds or cell lines), oral or any other form, financial and marketing plans, customer and supplier lists and information, and all other intellectual property or proprietary rights.

1.50 “JCC” has the meaning set forth in Section 4.2(a).

1.51 “JSC” has the meaning set forth in Section 4.1(a).

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1.52 “Losses” has the meaning set forth in Section 6.1.

1.53 “Lost Profit” has the meaning set forth in Section 2.6.

1.54 “Manufacture” and “Manufacturing” shall mean all activities related to the production, manufacture, processing, filling, finishing, packaging, labeling, shipping and holding of the Product or any intermediate thereof, including process development, process qualification and validation, scale up, pre-clinical, clinical and commercial manufacture and analytical development, product characterization, stability testing, quality assurance and quality control. When used as a verb, “Manufacture” shall mean to engage in Manufacturing.

1.55 “Marketing Authorization” shall mean the authorization issued by the relevant Regulatory Authority necessary to place on the market the Product in any country or regulatory jurisdiction in the Territory (including the centralized approval of a Marketing Authorization Application in the EU). For clarity, a Marketing Authorization shall not include any applicable Price and Reimbursement Approvals.

1.56 “Marketing Authorization Application” shall mean an application submitted to a Regulatory Authority for marketing approval of a drug or biologic product, including (a) a Marketing Authorization Application in the EU under Regulation (EC) No. 726/2004 or Directive 2001/83/EC, (b) any non-EU equivalent of the foregoing in any other country in the Territory, and (c) all supplements and amendments that may be filed with respect to any of the foregoing.

1.57 “Marketing Plan” has the meaning set forth in Schedule 8.1(a).

1.58 “Minimum FTEs” has the meaning set forth in Schedule 8.1(a).

1.59 “Minimum Order Quantity” has the meaning set forth in Section 2.4(d).

1.60 “Net Sales” shall mean the total amount of invoiced sales of the Product in the Territory by or on behalf of Chiesi or its Affiliates or Sub-distributors to Third Parties (including wholesalers, hospitals, end users and others), in bona fide arm’s length transactions, less the following deductions, in each case related specifically to the Product and customary in the trade and actually allowed and taken by such Third Parties and not otherwise recovered by or reimbursed to Chiesi: (a) cash discounts allowed and actually taken; (b) taxes on sales (such as sales or use taxes) to the extent added to the sale price and set forth separately as such in the total amount invoiced; (c) freight and insurance to the extent added to the sale price and set forth separately as such in the total amount invoiced; (d) amounts repaid or credited by reason of rejections, defects, recalls, expirations, or returns; and (e) any governmental mandated charge backs, rebates, and discounts. No deductions shall be made for (x) commissions paid to individuals, whether they are with independent sales agencies or regularly employed by Chiesi or any of its Affiliates, and on its payroll, (y) the cost of collections, and (z) any advertising and promotional expenses. In no event shall Chiesi have a right to apply any discounts or deductions on the Product, resulting from Chiesi entering into “package deals” whereby Chiesi sells more than one product (in addition to the Product) to a customer and offers “package deal discounts”.

1.61 “Non-Controlling Party” has the meaning set forth in Section 7.5(b).

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1.62 “Party” and “Parties” has the meaning set forth in the first and opening paragraph of this Agreement.

1.63 “Patents” shall mean any patent or patent application, including utility patents, utility models, design patents, provisional applications, certificates of invention, and all divisionals, continuations, continuations-in-part, substitutions, reissues, reexaminations, renewals, extensions (including any supplemental protection certificate) or additions to any patent or patent application that Cover the Product owned or Controlled by either of the Parties as of the Effective Date or during the Term. Schedule 1.63 sets forth a list of Patents that Cover the Product in the Territory owned or Controlled by uniQure as of the Effective Date, such list to be updated or confirmed upon the date this Agreement has become effective pursuant to Section 9.1(b).

1.64 “Person” shall mean any natural person or any corporation, company, partnership, limited liability company, joint venture, firm, agency or other entity, including a Party.

1.65 “Price and Reimbursement Approval” shall mean any approval or authorization of any Regulatory Authority establishing a pricing- and payment scheme or a reimbursement scheme for the Product in any country or jurisdiction of the Territory.

1.66 “Product” shall mean the medicinal product set forth in Schedule 1.66, and any Improvements thereof.

1.67 “Product Complaint” shall mean any oral or written communication of dissatisfaction issued by any Regulatory Authority regarding the identity, quality, durability, reliability or performance of any Product, including appearance, low fills, foreign materials, foreign product, defective packaging or defective labeling.

1.68 “Profit” has the meaning set forth in Section 2.6.

1.69 “Publishing Party” has the meaning set forth in Section 10.5(a).

1.70 “Purchase Price” has the meaning set forth in Section 2.3(b).

1.71 “Quality Agreement” has the meaning set forth in Section 3.3(a).

1.72 “Registry” shall mean an organized system that uses observational study methods to collect uniform data (clinical and other) to evaluate specified outcomes for a population defined by a particular disease, condition, or exposure, and that serves one or more predetermined scientific, clinical, or policy purposes and meets the requirements of the EMA.

1.73 “Regulatory Approval” shall mean any and all approvals (including, where required, any applicable Price and Reimbursement Approvals), licenses, registrations or authorizations of any Regulatory Authority necessary for the Commercialization or use of a Product in a country or jurisdiction, including Marketing Authorizations.

1.74 “Regulatory Authority” shall mean any federal, national, multinational, state, provincial or local regulatory agency, department, bureau or other governmental entity with authority over the testing, Regulatory Approval, manufacture, use, storage,

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import, promotion, marketing or sale of a drug or biologic product in a country or jurisdiction, including the EMA.

1.75 “Regulatory Plan” has the meaning set forth in Section 3.2.

1.76 “Required Manufacturing Changes” has the meaning set forth in Section 3.4(a).

1.77 “SDEA” has the meaning set forth in Section 3.3(b).

1.78 “Specifications” shall mean the composition, quality and specifications regarding the Product as may be amended, modified or supplemented from time to time in accordance with the terms hereof. The initial Specifications are annexed as Schedule 1.78.

1.79 “Sub-distributor” shall mean a Third Party that is granted a sub-distribution or other Commercialization right in the Territory by Chiesi in accordance with this Agreement.

1.80 “Subject Disclosure” has the meaning set forth in Section 10.3(b).

1.81 “Target Price” has the meaning set forth in Schedule 8.1(a).

1.82 “Term” has the meaning set forth in Section 9.1(a).

1.83 “Territory” shall mean (i) the EU Member States, Iceland, Liechtenstein and Norway and (ii) Albania, Andorra, Bosnia, Croatia, Macedonia, Monaco, Montenegro, Republic of San Marino, Serbia (including Kosovo), Switzerland and Vatican City ((i) and (ii), collectively, “Territory A”) as well as (iii) Algeria, Brazil, China, Egypt, Mexico, Morocco, Pakistan, Russia and ex-CIS countries (i.e. Armenia, Azerbaijan, Belarus, Georgia, Kazakhstan, Kirghizstan, Moldova, Tajikistan, Turkmenistan, Ukraine and Uzbekistan), Tunisia and Turkey (“Territory B”).

1.84 “Territory A” has the meaning set forth in Section 1.83.

1.85 “Territory B” has the meaning set forth in Section 1.83.

1.86 “Third Party” shall mean any Person other than uniQure, Chiesi, or their respective Affiliates.

1.87 “Trademark” has the meaning set forth in Section 2.2(a)(i).

1.88 “uniQure” has the meaning set forth in the first and opening paragraph of this Agreement.

1.89 “uniQure Intellectual Property Rights” shall mean all Intellectual Property Rights Controlled by uniQure on the Effective Date or during the Term which would be infringed by the Commercialization of the Product as provided for in this Agreement.

1.90 “Valid Claim” shall mean any claim within an issued and unexpired Patent or within a pending Patent application that (i) is not expired, lapsed, or abandoned, (ii) is not dedicated to the public, disclaimed, or admitted to be unenforceable or invalid; and (iii) has not been invalidated, held unenforceable or cancelled by a court or administrative

agency of competent jurisdiction in an order or decision from which no appeal has been or can be taken, including through opposition, re-examination, reissue, disclaimer or otherwise.

1.91 “Interpretation”. Any reference in this Agreement to an Article, Section, subsection, paragraph, clause, or Schedule shall be deemed to be a reference to any Article, Section, subsection, paragraph, clause, or Schedule, of or to, as the case may be, this Agreement. Except where the context clearly otherwise requires, (a) wherever used, the use of any gender will be applicable to all genders, (b) the singular shall include the plural and vice versa, (c) any definition of or reference to any agreement, instrument or other document refers to such agreement, instrument other document as from time to time amended, supplemented or otherwise modified (subject to any restrictions on such amendments, supplements or modifications set forth herein or therein), (d) any reference to any Applicable Laws refers to such Applicable Laws as from time to time enacted, repealed or amended, (e) the words “herein”, “hereof” and “hereunder”, and words of similar import, refer to this Agreement in its entirety and not to any particular provision hereof, (f) the words “include”, “includes” and “including” are deemed to be followed by the phrase “but not limited to”, “without limitation” or words of similar import, (g) the word “or” has the inclusive meaning (i.e., “and/or”), (h) the word “day” means a calendar day, the word “month” means a calendar month, and the word “year” means, and the word “annual” refers to, a calendar year, (i) the word “quarterly” refers to a calendar quarter, (j) each accounting term used herein that is not specifically defined herein has the meaning given to it under the International Financial Reporting Standards, and (k) the captions or headings of the Schedule, Articles, Sections or other subdivisions hereof are inserted only as a matter of convenience or for reference and shall have no effect on the meaning of the provisions hereof.

## ARTICLE II APPOINTMENT, SUPPLY OF PRODUCTS

### 2.1 Appointment, Consideration.

(a) Subject to the terms hereof, uniQure hereby appoints Chiesi as its distributor with the exclusive right to Commercialize the Product solely in the Field in the Territory during the Term.

(b) Chiesi shall be entitled to appoint any of its Affiliates or, subject to the prior written consent of uniQure, which shall not be unreasonably withheld, Third Parties as sub-distributor to the extent required for Commercialization of the Product in the Field in the Territory. In this event, Chiesi shall procure, and shall remain ultimately responsible for, compliance by such Affiliates or Sub-distributors with all the relevant obligations of Chiesi hereunder.

(c)(1) Subject to the condition precedent pursuant to Section 9.1(b), Chiesi shall pay to uniQure, after receipt of a proper invoice, a one-time, non-refundable fee of EUR 2,000,000.00 (in words: two million Euro) in recognition of uniQure’s past expenditure developing the Product, within [\*\*] Business Days after this Agreement has become effective pursuant to Section 9.1(b).

(c)(2) In consideration of the licenses, rights and interest granted under this Agreement, and in addition to any other payments due hereunder, Chiesi shall pay to

uniQure, after receipt of a proper invoice, the following commercial milestone payments, in each case within [\*\*] days after the end of the corresponding calendar year:

- (A) EUR [\*\*] (in words: [\*\*] Euro) when cumulated Net Sales of the Product achieve EUR [\*\*] (in words: [\*\*] Euro) in a calendar year;

- (B) EUR [\*\*] (in words: [\*\*] Euro) when cumulated Net Sales of the Product achieve EUR [\*\*] (in words: [\*\*] Euro) in a calendar year;
- (C) EUR [\*\*] (in words: [\*\*] Euro) when cumulated Net Sales of the Product achieve EUR [\*\*] (in words: [\*\*] Euro) in a calendar year;
- (D) EUR [\*\*] (in words: [\*\*] Euro) when cumulated Net Sales of the Product achieve EUR [\*\*] (in words: [\*\*] Euro) in a calendar year.

Within [\*\*] days after the end of each calendar year, Chiesi shall inform uniQure of the occurrence or, as the case may be, non-occurrence, of any such milestone event.

For the avoidance of doubt, each milestone is payable once, up to a maximum of EUR 42,000,000.00 (in words: forty two million Euro) in milestone payments and only one milestone is payable for any given calendar year. For clarity, the highest unpaid payment possible shall be paid with respect to a particular calendar year, and any payment not made because a higher payment was due shall be available for payment if the relevant Net Sales threshold is achieved in a subsequent year. For example, if, in calendar year 2014, Product sales of EUR [\*\*] (in words: [\*\*] Euro) are first achieved and Product sales had not achieved EUR [\*\*] (in words: [\*\*] Euro) in calendar year 2013, then EUR [\*\*] (in words: [\*\*] Euro) shall be due with respect to the sales in 2014 and if, in 2015, Product sales achieve at least EUR [\*\*] (in words: [\*\*] Euro), but less than EUR [\*\*] (in words: [\*\*] Euro), then the EUR [\*\*] (in words: [\*\*] Euro) payment shall be due with respect to the sales in 2015.

(d) Chiesi shall keep complete and accurate records of Product sold or otherwise made available as appropriate to determine the amount of commercial milestones and other payments to be paid to uniQure. These records shall be retained for at least [\*\*] years after delivery of the Product pursuant to Section 2.5. uniQure shall have the right [\*\*] at uniQure's expense to retain an independent certified public accountant selected by uniQure, and reasonably acceptable to Chiesi, to review such records in the location(s) where such records are maintained by Chiesi upon reasonable notice and during regular business hours and under obligations of confidence. Results of such review shall be made available to both Parties. If the review indicates that there was an underpayment of any amount payable to uniQure, the amount of such underpayment shall be remitted to uniQure within [\*\*] days after such review, together with interest calculated in the manner provided in paragraph (f) below. If the underpayment is equal to or greater than [\*\*] percent ([\*\*]%) of the amount that was otherwise due, Chiesi shall pay all of uniQure's reasonable out-of-pocket expenses of such review. If the review

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indicates that there was an overpayment of any amounts by Chiesi, Chiesi may apply the amount of such overpayment to any future payment due to uniQure under Section 2.3.

(e) All payments to be made under this Agreement shall be made in EUR by wire transfer to the account designated by uniQure in writing. If amounts (e.g. Average Net Sales Price or Fully Loaded Cost of Goods) relevant for the calculation of any payments to be made under this Agreement are in a currency other than Euros, such amounts shall be expressed in their Euro equivalent, calculated on the last Business Day of the calendar months to which the applicable amounts relate using the currency converter at [www.oanda.com](http://www.oanda.com). Unless otherwise expressly set forth herein, all payments to uniQure are to be executed without any further deduction within [\*\*] days after receipt by Chiesi of the invoice with respect thereto.

(f) Any payment to uniQure under this Agreement that is not paid on or before the date such payment is due shall bear interest at the lesser of (i) [\*\*] per year, or (ii) the highest rate permitted by Applicable Laws, calculated on the number of days such payments are overdue.

(g) Any payment to be made under this Agreement shall be made plus value-added tax, if applicable.

(h) To the extent that any payments hereunder by Chiesi to uniQure are subject to tax, Chiesi shall pay such tax; provided, however, that, with respect to any payments subject to withholding tax, Chiesi shall pay the applicable withholding tax amount to the relevant taxing authority and promptly provide uniQure with all necessary documentation for uniQure to recover such tax. Chiesi will take all reasonable and lawful steps to minimize the amount of any such withholding tax obligation and uniQure shall promptly provide all information and documentation in its possession necessary for doing so.

## 2.2 Trademark, Labeling.

### (a) *Trademark.*

(i) The Product will be Commercialized by Chiesi in the Field in the Territory exclusively under the trademark "Glybera" (as defined in Schedule 2.2(a)) or, subject to the prior written consent of uniQure, such alternative trademark identified by Chiesi (the "Trademark"). In the event that Chiesi provides sufficient written evidence to uniQure that the use of an alternative trademark is required under Applicable Laws to lawfully Commercialize the Product in any country or jurisdiction of the Territory and if Chiesi identifies any trademark other than "Glybera" for this purpose, then Chiesi shall be entitled to Commercialize the Product under such alternative trademark without the prior written consent of uniQure. In the event that Chiesi identifies any trademark other than "Glybera" for other material commercial reasons, Chiesi shall provide sufficient written evidence for such reasons to uniQure and shall not be entitled to Commercialize the Product under an alternative Trademark without the prior written consent of uniQure, such consent not to be unreasonably withheld. Chiesi shall inform uniQure promptly of the need of such alternative trademark, such notice to be accompanied by the aforementioned written evidence and a list of at least [\*\*] alternative trademarks identified by Chiesi and suitable for Commercialization of the Product throughout the entire Territory.

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(ii) In case the Product is Commercialized by Chiesi under the Trademark "Glybera", uniQure hereby grants to Chiesi the exclusive, royalty-free, perpetual, irrevocable, right and license (subject to Section 9.3 below) to use the Trademark "Glybera" to Commercialize the Product solely in the Field in the Territory, with the right to grant sublicenses to Sub-distributors according to Section 2.1(b). Further, uniQure hereby grants to Chiesi the non-exclusive, royalty-free, right and license to use uniQure's trade name (as defined in Schedule 2.2(a)) in each country of the Territory during the Term solely for the purpose of identifying uniQure as the manufacturer and Marketing Authorization holder of the Product as contemplated in this Agreement.

(iii) Chiesi acknowledges that, subject to the foregoing licenses, uniQure shall own all right, title and interest in and to the Trademark "Glybera" inside and outside the Field, whether inside or outside of the Territory as well as any goodwill associated with the Trademark "Glybera". Chiesi shall ensure appropriate use of the trademark "Glybera" at all times in the entire Territory and observe the applicable trademark use guidelines issued by

uniQure, as amended from time, attached in Schedule 2.2(b). Chiesi shall not, during the Term or thereafter, register, use, or attempt to obtain any right in and to (A) the trademarks “Glybera” and “uniQure”, or (B) any name, logo or trademark confusingly similar thereto. If Chiesi or any of its Affiliates or Sub-distributors challenges the validity of any such trademark during the Term, uniQure may terminate this Agreement in accordance with the provisions of Section 9.2(d). uniQure undertakes to maintain and defend the Trademark “Glybera” in each country inside the Territory during and, for as long as Chiesi retains licenses thereto, after the Term at its own cost. In the event that at any time during such term uniQure intends not to continue prosecution or maintenance of such Trademark anywhere inside the Territory, it shall inform Chiesi at least [\*\*] days prior to doing so and shall, upon request of Chiesi transfer all right, title and interest in such Trademark in such country or jurisdiction to Chiesi for further prosecution and maintenance by Chiesi in Chiesi’s name and at Chiesi’s costs and Chiesi shall reimburse uniQure for any reasonable external costs incurred by uniQure for such transfer.

(iv) uniQure acknowledges that except as otherwise expressly provided in this Agreement, Chiesi shall own all right, title and interest in and to any Trademark other than the trademark “Glybera” as well as any goodwill associated therewith. In case the Product is Commercialized by Chiesi under such alternative Trademark, Chiesi hereby grants to uniQure an exclusive, royalty-free, perpetual, irrevocable, right and license (subject to Section 9.3 below) to use such Trademark to Manufacture and Commercialize the Product outside the Territory, with the right to grant sublicenses. uniQure shall not, during the Term or thereafter, register, use, or attempt to obtain any right in and to (A) such Trademark and the “Chiesi” trademark, or (B) any name, logo or trademark confusingly similar thereto. If uniQure or any of its Affiliates challenges the validity of any such trademark during the Term, Chiesi may terminate this Agreement in accordance with the provisions of Section 9.2(d). Chiesi undertakes to obtain, maintain and defend such Trademark in each country inside and, as requested by uniQure, outside of the Territory during and, for as long as uniQure retains licenses thereto, after the Term at its own cost. In the event that at any time during such term Chiesi intends not to continue prosecution or maintenance of such Trademark anywhere inside or outside of the Territory it shall inform uniQure at least [\*\*] days prior to doing so and shall, upon request of uniQure transfer all right, title and interest in such Trademark in such country or jurisdiction to uniQure for further prosecution and

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maintenance by uniQure in uniQure’s name and at uniQure’s costs and uniQure shall reimburse Chiesi for any reasonable external costs incurred by Chiesi for such transfer.

(b) *Labeling.*

(i) uniQure as Marketing Authorization holder of the Product in Territory A shall be ultimately responsible for the content and type of all labeling and packaging (and any changes or supplements thereto) for the Product to be Commercialized by Chiesi in the Field in Territory A. Notwithstanding the foregoing, the Parties agree that, subject to Section 3.1, (A) Chiesi shall provide all reasonable assistance to uniQure in connection with the labeling and packaging for the Product (e.g. use of Chiesi in-house capacity for the translation of package leaflets), and (B) the Product to be Commercialized by Chiesi in the Field in Territory A shall include, to the extent legally permitted, a reference to Chiesi as Commercialization partner, and shall take into account, to the extent legally permitted, Chiesi’s livery. Details shall be agreed upon between the Parties in the Regulatory Plan. Chiesi shall take out at its costs any necessary insurance required under Applicable Laws as a result of Chiesi being referred to as a Commercialization partner on the Product, and shall reimburse uniQure for any costs associated with changes to the labeling and packaging of the Product in accordance with the foregoing.

(ii) Chiesi as Marketing Authorization holder of the Product in Territory B shall be ultimately responsible for the content and type of all labeling and packaging (and any changes or supplements thereto) for the Product to be Commercialized by Chiesi in the Field in Territory B. Notwithstanding the foregoing, the Parties agree that, subject to Section 3.1, (A) uniQure shall provide all reasonable assistance to Chiesi in connection with the labeling and packaging for the Product, and (B) the Product to be Commercialized by Chiesi in the Field in Territory B shall include, to the extent legally permitted, a reference to uniQure as originator and manufacturer of the Product, and shall take into account, to the extent legally permitted, uniQure’s livery. Details shall be agreed upon between the Parties in the Regulatory Plan. uniQure shall take out at its costs any necessary insurance required under Applicable Laws as a result of uniQure being referred to as originator and manufacturer on the Product, and shall reimburse Chiesi for any costs associated with changes to the labeling and packaging of the Product in accordance with the foregoing.

(iii) If, according to local mandatory regulatory requirements, uniQure is not eligible as Marketing Authorization holder of the Product in any country of Territory A, or Chiesi is not eligible as Marketing Authorization holder of the Product in any country of Territory B, Chiesi or, as the case may be, its Sub-distributor, or uniQure, shall become the Marketing Authorization holder of the Product in such country of the Territory. In such case, Chiesi or, as the case may be, its Sub-distributor, or uniQure, shall be ultimately responsible for the content and type of all labeling and packaging (and any changes or supplements thereto) for the Product to be Commercialized by Chiesi in the Field in such country of the Territory, and sentences 2 to 4 of paragraph (i) or (ii) above shall apply, respectively.

2.3 Purchase of the Product.

(a) *Orders.* During the Term, Chiesi shall purchase from uniQure one hundred percent (100%) of Chiesi’s requirements for the Product for Commercialization

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in the Field in the Territory. Purchase shall be made pursuant to Firm Orders submitted by Chiesi to uniQure from time to time in accordance with Section 2.4.

(b) *Purchase Price.* The purchase price for the individual Product ordered shall be the greater of (i) [\*\*] and (ii) [\*\*] (the “Purchase Price”).

(c) *Invoicing, Payment.* uniQure shall invoice Chiesi for all quantities of Product as follows:

(i) As upfront payment to the Purchase Price, Chiesi shall pay to uniQure the Fully Loaded Cost of Goods, reduced by the cost items incurred by uniQure only after receipt of the corresponding Delivery Notification as identified in Schedule 2.3, for each patient dose of the Product delivered (i.e. through storage at uniQure’s warehouse) in accordance with Section 2.4 (“Glybera Manufacturing Cost Reimbursement”). uniQure shall promptly inform Chiesi of the occurrence of each such event.

(ii) Chiesi shall pay the difference between the Purchase Price and the Glybera Manufacturing Cost Reimbursement within [\*\*] days following the delivery of a particular patient dose of the Product in accordance with Section 2.5.

(iii) Section 2.1(e) to (h) shall apply.

#### 2.4 Forecasts; Firm Orders.

(a) *Forecasts.* Chiesi shall provide uniQure a non-binding forecast by [\*\*], detailing the quantity of Product required for 2013. Every [\*\*] months thereafter (i.e. no later than [\*\*]) Chiesi shall provide uniQure a non-binding forecast detailing the quantity of Product per [\*\*] required for the respective following [\*\*] months period. Chiesi shall make all forecasts in good faith given market and other information available to Chiesi.

(b) *Firm Orders.* Chiesi shall purchase the Product solely by Firm Orders. Firm Orders consist of the number of patient doses for a period of [\*\*] months specified per month. Chiesi shall submit each such Firm Order to uniQure at least [\*\*] months in advance of the anticipated release date as specified in each Firm Order. Chiesi shall submit Firm Orders for the Product [\*\*] times per calendar year no later than [\*\*]. Notwithstanding the foregoing and the condition precedent set forth in Section 9.1(b), Chiesi shall submit the first Firm Order no later than [\*\*] days after the Effective Date. Any terms or conditions contained in any Firm Order, acknowledgment, invoice, bill of lading, acceptance, or other writing or document issued by either Party, whether or not in conflict with the terms of this Agreement, shall be null and void without further notice required to be given by the other Party.

(c) *Order Processing.* In order to become effective, each Firm Order placed by Chiesi shall be confirmed by uniQure by facsimile or email showing the confirmed quantity indicated in each Firm Order and delivery (i.e. through storage at uniQure's warehouse) date of the Product within [\*\*] Business Days of receipt (the "Confirmed Firm Order"), provided that, if uniQure does not provide an acknowledgment of the Confirmed Firm Order within such period, uniQure shall be deemed to have confirmed the corresponding Firm Order. uniQure shall not be obliged to accept and

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fulfill any Firm Orders which exceed [\*\*] percent ([\*\*]%) of the Product quantity indicated in each relevant forecast. uniQure shall ensure availability of confirmed quantities of the Product within [\*\*] months after uniQure's receipt of the corresponding Firm Order, provided, however, that in case a Firm Order exceeds [\*\*] percent ([\*\*]%) of the Product quantity indicated in each relevant forecast for the relevant period, and uniQure accepts such Firm Order, uniQure and Chiesi shall agree to a new release date and uniQure shall be entitled to release any amounts of Product exceeding the above threshold within [\*\*] months after uniQure's receipt of such Firm Order. uniQure shall store such Product at uniQure's warehouse at uniQure's cost.

(d) *Batch Sizes.* All quantities of Product ordered by Chiesi shall be consistent with uniQure's current minimum batch sizes for the applicable Product (the "Minimum Order Quantity"), or multiples thereof, as set forth in Schedule 2.4(d). Notwithstanding the foregoing, uniQure agrees to support and cooperate with Chiesi to accept, fulfill and deliver order quantities at amounts less than the Minimum Order Quantity, in the event that Chiesi's good faith internal evaluations or the requirements of customers for the Product support or require smaller quantity Firm Orders.

#### 2.5 Shipment and Delivery.

(a) *Terms.* Within [\*\*] Business Days following receipt of a Delivery Notification from Chiesi, UniQure shall manufacture the finished dosage form of the Product for the quantities specified in the Delivery Notification (not to exceed the quantities in the respective Confirmed Firm Order), and ship such quantities of Product directly to the customers of Chiesi, together with a corresponding invoice to Chiesi. The Parties shall agree, after [\*\*] the Effective Date, on a reduction of the [\*\*] Business Day period set forth above. All quantities of Product shall be delivered EXW uniQure's facility in Amsterdam. Chiesi shall obtain at its cost all necessary export or import licenses and permits to export or import the relevant quantities of the Product into the relevant country or jurisdiction of the Territory. Title and risk of loss and damage for any Product delivered pursuant to this Agreement shall pass to Chiesi at the time the same are tendered by uniQure to the carrier for delivery to Chiesi's customers. uniQure shall pack Product for shipment in accordance with uniQure's standard procedures and Applicable Laws, unless otherwise specified in writing by Chiesi within the scope of mandatory Applicable Laws [\*\*] days prior to such shipment, in which event any extra costs incurred by uniQure on account of the packaging changes requested by Chiesi shall be promptly reimbursed by Chiesi.

(b) *Delivery Notification.* For each supply of Product to each of its customers, Chiesi shall ensure and confirm to uniQure in writing (each a "Delivery Notification") that (i) the Product has obtained Price and Reimbursement Approval in the relevant part of the Territory, except for Germany until the relevant Price and Reimbursement Approval has been obtained, and (ii) the healthcare professionals involved in the treatment of a patient have received the educational pack and the patient to be treated with the supplied Product is included in the Registry.

(c) *Release.* uniQure shall perform release testing pursuant to the Specifications and in accordance with uniQure's standard procedures regarding the Manufacturing of Product. After each shipment of Product, uniQure shall release the Product and provide to Chiesi a Certificate of Analysis and a Certificate of Compliance

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and such other documents as may be required by Applicable Laws or mutually agreed by the Parties.

(d) *Minimum Remaining Shelf-life and Returns.* Product returns shall be the sole responsibility of Chiesi and uniQure shall have no obligation with respect to any such returned Product, provided that the releasing Qualified Person should be informed of return and final disposition and all Products supplied by uniQure hereunder shall have a minimum remaining shelf life upon delivery equal to no less than [\*\*] months of the shelf-life set forth in the relevant Specifications or such longer period as required by a relevant customer in the Territory.

#### 2.6 Failure to Supply.

(a) In the event that it becomes apparent to uniQure that it will be unable to fulfill any Confirmed Firm Order for the Product ("Failure to Supply"), uniQure shall, immediately after learning of such event or circumstances, notify Chiesi in writing of uniQure's Failure to Supply, along with a reasonable explanation of the reason, to the extent then known to uniQure, for uniQure's Failure to Supply and with a specific indication of the quantity of Product affected by such Failure to Supply and anticipated timing of delivery of the Product. Promptly after Chiesi's receipt of any such notice, the Parties shall agree upon mutually acceptable revised quantities and delivery dates with respect to the Product subject to such Confirmed Firm Order or, to the extent this is not possible in light of the specific or then unknown reason for uniQure's Failure to Supply, shall discuss in good faith measures to further investigate the root cause and, as the case may be, appropriate steps to overcome such Failure to Supply.

(b) Notwithstanding paragraph (a), in the event that Chiesi cannot fulfill any firm orders for the Product received from any Third Parties as a consequence of uniQure's Failure to Supply, except if such Failure to Supply is caused as a result of any Force Majeure Event, then Chiesi shall be entitled to an indemnification payment equal to Chiesi's Lost Profit for the period during which Chiesi has been affected by the Failure to Supply. Any indemnification payment made to Chiesi under this paragraph for Failure to Supply shall be reimbursed in full to uniQure, in case any patient who suffered from the Failure to Supply is then subsequently treated. Such indemnification payments and reimbursements, if any, shall be calculated on a calendar year basis, such calculation to be made within [\*\*] days after the end of the corresponding calendar year and any resulting amount to be paid within [\*\*] days after such calculation has been made. uniQure, in relying on the above Force Majeure Event exceptions, shall provide reasonably detailed particulars of the reasons underlying any such Force Majeure Event to Chiesi and shall allocate its existing stocks of the Product between uniQure, its Affiliates, Chiesi and other distributors of the Product, on a pro-rata basis, based upon order volumes for the Product for the prior [\*\*]month period.

(c) For the purpose of this Section 2.6, "Profit" shall be calculated, on a per Product basis, as the difference between (a) the relevant Average Net Sales Price that would have applied to the Product affected by the Failure to Supply and (b) the applicable Purchase Price for the Product affected by the Failure to Supply calculated as per Section 2.3 above, and "Lost Profit" shall mean the accumulated Profit for all quantities of Product affected by the Failure to Supply.

(d) Without prejudice to the foregoing paragraphs (a) to (c), if

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(i) uniQure's Failure to Supply affects at least [\*\*] consecutive Confirmed Firm Orders for a period of no less than nine (9) months, and

(ii) the reason for uniQure's Failure to Supply could be established during the Parties' discussion pursuant to paragraph (a) above, and such reason was specifically related to uniQure's ability to Manufacture the Product at its current manufacturing site (i.e. the Failure to Supply could reasonably be expected to be overcome if the Product was Manufactured at a different manufacturing site),

upon either Party's request, the Manufacturing of the Product shall be transferred to (A) uniQure's US manufacturing site, provided such site is operational at the relevant point in time, and further provided uniQure, within [\*\*] following such request, does not opt against such transfer, and (B) otherwise (i.e. if uniQure opts against such transfer within the foregoing [\*\*] period) to any other Third Party manufacturer mutually agreed to by uniQure and Chiesi. uniQure shall efficiently and promptly transfer to its US manufacturing site or, as the case may be, such Third Party manufacturer all information, licenses and rights controlled by uniQure and necessary to Manufacture and supply the Product to Chiesi hereunder during the continuance of uniQure's Failure to Supply. Such transfer shall ensure uniQure's ongoing control over the information, licenses and right so transferred, shall include the steps outlined in Schedule 2.6, and shall occur through email and videoconference interactions, as well as face-to-face meetings as required to ensure efficient transfer of technologies and capabilities.

If uniQure's US manufacturing site or, as the case may be, such Third Party manufacturer is unable to Manufacture the Product within [\*\*] months after uniQure has started the technology transfer to such person, Chiesi shall have the right to terminate this Agreement with three (3) month notice in writing, except if uniQure's Failure to Supply is caused as a result of a Force Majeure Event. Such termination shall not become effective if, during such three (3) month notice period, uniQure has notified Chiesi of the ability of its US manufacturing site or, as the case may be, such Third Party manufacturer to Manufacture the Product. Upon termination of this Agreement by Chiesi pursuant to this Section 2.6(d), the provisions of Section 9.3(b) (i), (ii) and (iv) shall apply.

(e) Without prejudice to the foregoing paragraphs (a) to (c), if

(i) uniQure's Failure to Supply affects at least [\*\*] consecutive Confirmed Firm Orders for a period of no less than nine (9) months, and

(ii) the reason for uniQure's Failure to Supply (A) could be established during the Parties' discussion pursuant to paragraph (a) above, but such reason was not specifically related to uniQure's ability to Manufacture the Product at its current manufacturing site (i.e. the Failure to Supply could not reasonably be expected to be overcome if the Product was Manufactured at a different manufacturing site), or (B) could not be established during the Parties' discussion pursuant to paragraph (a) above during at least the foregoing nine (9) months period, and

(iii) uniQure's Failure to Supply is not caused as a result of a Force Majeure Event,

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Chiesi may terminate this Agreement with three (3) month notice in writing. Such termination shall not become effective if, during such three (3) month notice period, uniQure has notified Chiesi of the end of its Failure to Supply and has provided to Chiesi at least [\*\*] of the outstanding Confirmed Firm Orders. Upon termination of this Agreement by Chiesi pursuant to this Section 2.6(e), the provisions of Section 9.3(b) (i), (ii) and (iv) shall apply.

(f) Without prejudice to the foregoing paragraphs (a) to (e), if within [\*\*] months after uniQure has notified Chiesi in writing of uniQure's Failure to Supply, any Person is unable to Manufacture and supply the Product to Chiesi hereunder, then for any possible future supply of the Product to Chiesi hereunder, the percentage set out in Section 2.3(b)(i) above shall be reduced to [\*\*] percent ([\*\*]%) for any individual Product ordered after the end of uniQure's Failure to Supply for a period equivalent to the duration of uniQure's Failure to Supply (i.e. if uniQure's Failure to Supply lasted for [\*\*] months, the reduced percentage of [\*\*] percent ([\*\*]%) shall apply to any individual Product ordered during the [\*\*] months period after the end of uniQure's Failure to Supply).

### ARTICLE III QUALITY, REGULATORY AND PHARMACOVIGILANCE MATTERS

#### 3.1 Permits.

(a) uniQure shall be responsible for any filing, holding and maintenance associated with all Marketing Authorizations for the Product in Territory A, and Chiesi shall be responsible for any filing, holding and maintenance associated with all Marketing Authorizations for the Product in Territory B. Notwithstanding the foregoing, if, according to local mandatory regulatory requirements, uniQure is not eligible as Marketing Authorization holder of the Product in any country of Territory A, or Chiesi is not eligible as Marketing Authorization holder of the Product in any country of Territory B, Chiesi or, as the case may

be, its Sub-distributor, or uniQure, shall become the Marketing Authorization holder of the Product in such country of the Territory. Details regarding each Party's responsibilities and obligations and the exchange of information in the process of filing, holding and maintaining any Marketing Authorizations for the Product in the Territory shall be agreed upon between the Parties in the Regulatory Plan. Notwithstanding the foregoing and except as otherwise set forth in this Agreement, including the Regulatory Plan, the Quality Agreement and the SDEA, each Party shall, at such Party's sole cost and expense, maintain in full force and effect all other Regulatory Approvals required by Applicable Laws to carry out such Party's duties and obligations under this Agreement.

(b) Without prejudice to the generality of paragraph (a) above, (i) uniQure and Chiesi shall share equally the cost associated with the Registry in the EU, the PIP (Pediatric Investigation Plan) and any Phase IV clinical study regarding the Product mutually agreed between the Parties, (ii) uniQure shall be responsible for any filing, holding and maintenance fees associated with Marketing Authorizations and Marketing Authorization Applications in Territory A, including in the countries outside of the EU Member States, as further agreed between the Parties during the Term in accordance with Schedule 3.1, CMC (Chemistry, Manufacturing, and Controls), and pharmacovigilance regarding the Product in the Territory (except for the Registry), and (iii) Chiesi shall be responsible for any filing, holding and maintenance fees associated with Marketing

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Authorizations and Marketing Authorization Applications in Territory B as further agreed between the Parties during the Term in accordance with Schedule 3.1, any changes (other than Required Manufacturing Changes and Discretionary Manufacturing Changes which are governed by Section 3.4 below) Chiesi shall request with respect to the Product, and reporting of safety data regarding the Product in Territory B.

3.2 Regulatory Plan and Responsibility. The Parties shall adopt a regulatory plan relating to the Product (the "Regulatory Plan") a draft of which shall be attached as Schedule 3.2 and which shall be finalized by the Parties as soon as possible after the Effective Date, but in any event within [\*\*] weeks thereafter. The Regulatory Plan shall be approved by the JCC and may be updated from time to time through the JSC or the JCC.

3.3 Quality Agreement and Safety Data Exchange Agreement.

(a) As soon as possible after the Effective Date, but in any event within [\*\*] days after the Effective Date, the Parties shall enter into a quality agreement regarding the Product (the "Quality Agreement"), whereby the Parties shall define their respective responsibilities in relation to GDPs and quality matters, Specifications, release and supply of the Product. In the event of a conflict between the terms of this Agreement and the Quality Agreement, the terms of this Agreement shall govern.

(b) Without prejudice to the generality of Section 3.1(b) above, as soon as possible after the Effective Date, but in any event within [\*\*] days after the Effective Date, the Parties shall enter into a safety data exchange agreement (the "SDEA") that defines the roles and responsibilities of each Party in terms of pharmacovigilance and the detailed safety exchange required to permit compliance by each Party with safety reporting requirements to Regulatory Authorities in the Territory. In the event of a conflict between the terms of this Agreement and the SDEA, the terms of this Agreement shall govern.

3.4 Change Management.

(a) For changes to the Specifications or Manufacture processes of the Product that are required by Applicable Laws in the Territory (collectively, "Required Manufacturing Changes"), uniQure and Chiesi shall cooperate in making such changes in a timely manner.

(b) For changes to the Specifications or Manufacture processes of the Product that are not Required Manufacturing Changes (collectively, "Discretionary Manufacturing Changes"), uniQure and Chiesi must each agree in writing to any Discretionary Manufacturing Changes before such change is implemented, provided that neither Party shall unreasonably withhold its consent to such Discretionary Manufacturing Changes.

(c) Unless otherwise agreed between the Parties, through the JSC or JCC, uniQure's quality system, as further defined in the Quality Agreement, shall be utilized by the Parties in reviewing and implementing any changes under this Section 3.4.

(d) The commercially reasonable costs, including obsolete raw materials, work-in-process, product packaging and labeling materials, (i) associated with

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Required Manufacturing Changes shall be shared equally between uniQure and Chiesi, and (ii) associated with Discretionary Manufacturing Changes shall be borne by the Party initiating such changes.

3.5 Stability Testing; Validation. uniQure shall perform stability testing, process validation or cleaning validations with respect to the Product in accordance with uniQure's standard procedures, as further defined in the Quality Agreement, and Applicable Laws. Any additional testing reasonably requested by Chiesi shall be performed by uniQure at the expense of Chiesi.

3.6 Recalls; Product Complaints.

(a) uniQure shall have the sole authority and responsibility to respond to any Regulatory Authority, to respond to Product Complaints and to handle all recalls and market withdrawals of the Product in accordance with Applicable Laws, provided, that in all cases, unless otherwise required to comply with any Applicable Laws or any decision, order, request or directive of a Regulatory Authority, Chiesi shall release no communication into the marketplace regarding such Product Complaints, recalls or market withdrawals without first obtaining uniQure's consent to such communication, which shall not be unreasonably withheld. Other complaints related to the Product, in particular complaints not related to regulatory matters, shall be managed solely by Chiesi.

(b) Each Party shall promptly (but in any case, not later than [\*\*] Business Days) notify the other Party in writing of any decision, order, request or directive of a Regulatory Authority to recall, withdraw or field correct any Product. UniQure shall promptly (but in any case, not later than [\*\*] Business Days) notify Chiesi of any voluntary decision to recall, withdraw or field correct any Product. uniQure shall be solely responsible for determining whether to issue a recall, withdrawal, or field correction (but shall comply with all Applicable Laws in making such determination) and for the cost and expense of any such recall, withdrawal or field correction; provided, that uniQure shall give due consideration to all comments timely made by Chiesi relating to the Manufacture or testing of the Product and shall notify Chiesi in writing if uniQure declines to address any such comments, stating the reason therefor. If any recall, market withdrawal or field correction is not due to uniQure's Manufacture of the Product, then uniQure shall be relieved of uniQure's obligations to supply the



Product hereunder until the cause of such recall, withdrawal or field correction has been resolved to the satisfaction of the Parties and the applicable Regulatory Authority, and during such period such relief shall be deemed a Force Majeure Event for the purposes of this Agreement.

(c) Notwithstanding the foregoing paragraphs (a) and (b), but without prejudice to any obligations of uniQure under mandatory Applicable Laws, to the extent possible with view to any timelines applicable under Applicable Laws, the Parties, through the JSC or JCC, shall mutually discuss any of the foregoing events (i.e. response to any Regulatory Authority, response to Product Complaints, recalls, market withdrawals, field corrections) and agree on a joint communication in relation to such event both to any Regulatory Authority and the marketplace taking into account both the regulatory and commercial implications associated with such event and communication.

3.7 Notice of Government Inspections. Each Party agrees that, to the extent such Party becomes aware of the results, observations or outcome of any inspections or

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audits of the facilities or operations involved in the Manufacture or Commercialization of the Product conducted by a Regulatory Authority, such Party will notify the other Party of any such information as it directly relates to the Product within [\*\*] Business Days after obtaining the information and shall provide the other Party with a copy of any written materials provided by the Regulatory Authority in connection with such inspection or audit. Each Party will provide the other with copies of reports of quality audits conducted by such Party with respect to the Product and will apprise the other Party of material Manufacture, marketing, promotion, sales, or other issues affecting supply or Commercialization of the Product.

3.8 Government Inquiries. If either Party shall be contacted by any Regulatory Authority for any regulatory purpose pertaining specifically to this Agreement or to the Product, such Party shall immediately notify the other Party. Either Party may permit unannounced inspections of the Product or facilities by a Regulatory Authority with competent jurisdiction and may respond to the extent necessary to comply with such Party's obligations under Applicable Laws.

3.9 Inspections / Audit of Records and Facilities. Unless otherwise required by Applicable Laws or any decision, order, request or directive of a Regulatory Authority, [\*\*](or in case of uniQure's facilities being inspected, up to [\*\*] times a year [\*\*]), for a period of no more than [\*\*] Business Days and by no more than [\*\*] designated personnel, each Party shall have reasonable access during normal business hours to the other Party's regulatory files as they relate to the Manufacture and Commercialization of the Product in the Territory to (a) review all such records, correspondence, notices, documents, and other materials (including warning letters and letters of adverse findings) and (b) inspect the other Party's facilities for compliance with this Agreement, in particular to inspect and audit uniQure's standard procedures regarding the Manufacturing of Products. Any inspection shall not unreasonably disrupt the normal operations of the inspected Party and shall be announced with a notice period of at least [\*\*] months prior to such audit.

3.10 Price and Reimbursement Approvals. Subject to Section 8.1(a) and(c) below, taking into account uniQure's unique experience and understanding of Gene Therapy generally and the Product specifically, both Parties agree that Chiesi and uniQure shall jointly consult and prepare the pricing strategy for the Price and Reimbursement Approvals of the Product in the Field in the Territory to be filed and obtained by Chiesi. For the avoidance of doubt and in accordance with Section 8.1(c) below, such consultation shall not establish or create any obligation of Chiesi to set a certain or fixed price for the Product.

#### ARTICLE IV GOVERNANCE; DECISION MAKING

##### 4.1 Joint Steering Committee.

(a) *Formation and Membership.* Within [\*\*] days after the Effective Date, Chiesi and uniQure shall establish a joint steering committee (the "JSC") to manage the Collaboration. The JSC to be established under this Agreement shall be identical to the one to be established under the Co-Development and License Agreement. The JSC shall be comprised of [\*\*] executives or senior employees of Chiesi and [\*\*] executives or senior employees of uniQure with appropriate experience and level of decision-making

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authority. From time to time, in addition to the JCC described below, the Parties may establish one or more subcommittees of the JSC to oversee particular projects or activities (e.g., activities under the Co-Development and License Agreement, financial reporting). Each such subcommittee shall be comprised of an equal number of representatives from each Party with appropriate experience and level of decision-making authority. Each subcommittee shall meet with a frequency to be agreed on by the Parties. Each Party may change any one or more of its representatives on the JSC or any subcommittee at any time upon written notice to the other Party.

(b) *Responsibilities.* The JSC shall be responsible for:

- (i) providing overall direction of the Collaboration;
- (ii) attempting to resolve disputes arising under the Collaboration Agreements; and
- (iii) performing such other tasks and undertaking such other responsibilities as may be set forth in the Collaboration Agreements.

(c) *Meetings.*

(i) The JSC shall meet at least [\*\*], by tele- or video-conference or in person, with the meetings in approximately [\*\*] to be held in-person. The location of in-person JSC meetings shall alternate between the headquarters offices of each Party, with the first meeting to take place at [\*\*] within [\*\*] days after the Effective Date.

(ii) Each Party shall use reasonable efforts to cause its representatives to attend the meetings of the JSC and any subcommittees. In addition, each Party may, at its discretion, invite a reasonable number of non-voting employees or officers, and, with the consent of the other Party, consultants or scientific advisors, to attend meetings of the JSC or any subcommittee, or the relevant portion thereof; provided that, its representatives and any such other employees, officers, consultants or scientific advisors are bound by written obligations of confidentiality that are at least as stringent as those set forth in this Agreement. Each Party shall bear all travel and living expenses of its representatives and other employees, officers, consultants or scientific advisors incurred to attend the meetings of the JSC or any subcommittee.

(iii) Either Party may also request, by providing written notice to the other Party, that a special meeting of the JSC be convened for the purpose of resolving disputes in connection with, or for the purpose of reviewing or making a decision pertaining to, any material matter within the purview of the JSC, the examination or resolution of which cannot reasonably be postponed until the next scheduled JSC meeting. Such meeting shall be convened at such time as may be mutually agreed upon by the Parties, but in any event shall be held within [\*\*] days after the date of such notice.

(d) *Administrative Matters.* The right to appoint the chairperson of the JSC shall alternate on an annual basis between Chiesi and uniQure, with [\*\*] having the right to appoint the chairperson for the first year of the Term. The Alliance Managers shall work with the chairperson to develop JSC meeting agendas. The chairperson shall be responsible for calling meetings of the JSC and for leading the meetings. A JSC

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member of the chairing Party shall serve as secretary of such meetings. The secretary shall promptly prepare and distribute to all members of the JSC draft minutes of the meeting for review and comment, including a list of any actions or decisions approved by the JSC, with the goal of distributing final approved minutes of each JSC meeting within [\*\*] days after the meeting. Neither the chairperson nor the secretary shall have any greater authority than any other representative on the JSC and the Party appointing the chairperson and the secretary shall not have any greater authority than the other Party by virtue of its right to make such appointments. The chairperson shall include on the agenda any items proposed by either Party.

(e) *Decision Making.* Each Party, through its representatives, shall have one (1) vote on the JSC and each subcommittee. Both Parties must vote in the affirmative to allow the JSC or a subcommittee to take any action that requires the approval of the JSC or the subcommittee. Decision on any matter may be taken at a meeting, by teleconference, videoconference or by written agreement. If a subcommittee is unable to resolve any dispute, or to unanimously agree on any matter, within its responsibilities, such dispute or matter shall be referred to the JSC for resolution. Either Party may convene a special meeting of the JSC in accordance with Section 4.1(c)(iii) for the purpose of resolving any dispute within the JSC's jurisdiction that represents a material issue the resolution of which cannot reasonably await until the next scheduled meeting of the JSC.

(f) *Dispute Resolution by Executive Officers.*

(i) If the JSC is unable to resolve any dispute within the responsibilities of the JSC specified in Section 4.1(b) within [\*\*] days after a Party provides notice to the other Party of the existence of such dispute, such dispute or other matter shall be referred to the Executive Officers for resolution. If a dispute is referred to the Executive Officers for resolution pursuant to the preceding sentence, the Executive Officers shall attempt in good faith to resolve such dispute within [\*\*] days. In resolving any disputes under this Section 4.1(f), each Party shall act in good faith, subject to the terms and conditions of the Collaboration Agreements, and in a commercially reasonable manner without favoring other products being developed or commercialized by or on behalf of such Party or its Affiliates outside of the Collaboration.

(ii) If the Executive Officers are unable to reach a consensus in accordance with paragraph (i) above, (A) uniQure shall have final decision-making authority with respect to all matters related to research or development in relation to the Product, with reasonable input from Chiesi taking into account Territory-specific matters, (B) Chiesi shall have final decision-making authority with respect to all matters related to Commercialization of the Product in the Territory, with reasonable input from uniQure taking into account uniQure's global Product strategy, (C) subject to Sections 3.1 and 3.2, both Parties agree that on regulatory matters with respect to the Product they will jointly work towards a regulatory strategy for the Product in the countries of the Territory which are not EU Member States, and (D) any matter not falling within any of the foregoing categories (A) to (C) shall be decided by binding arbitration pursuant to Section 11.9 below.

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#### 4.2 Joint Commercialization Committee.

(a) *Formation and Membership.* Within [\*\*] days after the Effective Date, Chiesi and uniQure shall establish, as a subcommittee of the JSC, a joint commercialization committee (the "JCC") to manage the overall relationship between the Parties under this Agreement. The JCC shall be comprised of [\*\*] executives or senior employees of Chiesi and [\*\*] executives or senior employees of uniQure with appropriate experience and level of decision-making authority. From time to time, the Parties may establish one or more subcommittees of the JCC to oversee particular projects or activities (e.g., regulatory, supply, forecast, global brand integration). Each such subcommittee shall be comprised of an equal number of representatives from each Party with appropriate experience and level of decision-making authority. Each subcommittee shall meet with a frequency to be agreed on by the Parties. Each Party may change any one or more of its representatives on the JCC or any subcommittee at any time upon written notice to the other Party.

(b) *Responsibilities.* The JCC shall be responsible for:

(i) periodically reviewing the Regulatory Plan and suggesting or approving such updates or amendments to the Regulatory Plan as the JCC deems appropriate, including all budgets relating to activities to be conducted hereunder and amendments thereto;

(ii) ensuring consistency and coordination, to the maximum possible extent, between Commercialization activities to be conducted by uniQure in the Field outside the Territory and by Chiesi in the Field in the Territory;

(iii) providing overall strategic direction with respect to Commercialization and regulatory activities for the Product, including activities conducted under the Regulatory Plan;

(iv) overseeing Commercialization and regulatory activities for the Product;

(v) discussing and addressing any supply chain or other delivery issues that have arisen or might arise relating to the Product;

(vi) attempting to resolve disputes arising under this Agreement that are referred to the JCC by either Party or any subcommittee;

and

(vii) performing such other tasks and undertaking such other responsibilities as may be set forth in this Agreement or as may be delegated to it by the JCC.

(c) *Meetings.*

(i) The JCC shall meet at least [\*\*], by tele- or video-conference or in person, with the meetings in approximately [\*\*] to be held in-person. The location of in-person JCC meetings shall alternate between the headquarters offices of each Party, with the first meeting to take place at [\*\*] within [\*\*] days after the Effective Date.

(ii) Each Party shall use reasonable efforts to cause its representatives to attend the meetings of the JCC and any subcommittees.

In addition,

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each Party may, at its discretion, invite a reasonable number of non-voting employees or officers, and, with the consent of the other Party, consultants or scientific advisors, to attend meetings of the JCC or any subcommittee, or the relevant portion thereof; provided that, its representatives and any such other employees, officers, consultants or scientific advisors are bound by written obligations of confidentiality that are at least as stringent as those set forth in this Agreement. Each Party shall bear all travel and living expenses of its representatives and other employees, officers, consultants or scientific advisors incurred to attend the meetings of the JCC or any subcommittee.

(iii) Either Party may also request, by providing written notice to the other Party, that a special meeting of the JCC be convened for the purpose of resolving disputes in connection with, or for the purpose of reviewing or making a decision pertaining to, any material matter within the purview of the JCC, the examination or resolution of which cannot reasonably be postponed until the next scheduled JCC meeting. Such meeting shall be convened at such time as may be mutually agreed upon by the Parties, but in any event shall be held within [\*\*] days after the date of such notice.

(d) *Administrative Matters.* [\*\*] shall have the right to appoint the chairperson of the JCC. The Alliance Managers shall work with the chairperson to develop JCC meeting agendas. The chairperson shall be responsible for calling meetings of the JCC and for leading the meetings. A [\*\*] JCC member shall serve as secretary of such meetings. The secretary shall promptly prepare and distribute to all members of the JCC draft minutes of the meeting for review and comment, including a list of any actions or decisions approved by the JCC, with the goal of distributing final approved minutes of each JCC meeting within [\*\*] days after the meeting. Neither the chairperson nor the secretary shall have any greater authority than any other representative on the JCC and [\*\*] shall not have any greater authority than [\*\*] by virtue of its right to make such appointments. The chairperson shall include on the agenda any items proposed by either Party.

(e) *Decision Making.* Each Party, through its representatives, shall have one (1) vote on the JCC and each subcommittee. Both Parties must vote in the affirmative to allow the JCC or a subcommittee to take any action that requires the approval of the JCC or the subcommittee. Decision on any matter may be taken at a meeting, by teleconference, videoconference or by written agreement. If a subcommittee is unable to resolve any dispute, or to unanimously agree on any matter, within its responsibilities, such dispute or matter shall be referred to the JCC for resolution. Either Party may convene a special meeting of the JCC in accordance with Section 4.2(c)(iii) for the purpose of resolving any dispute within the JCC's jurisdiction that represents a material issue the resolution of which cannot reasonably await until the next scheduled meeting of the JCC.

(f) *Dispute Resolution.* If the JCC is unable to resolve any dispute within the responsibilities of the JCC specified in Section 4.2(b) within [\*\*] days after a Party provides notice to the other Party of the existence of such dispute, then, (A) the respective representative(s) of each Party in the JCC may exercise the final decision-making authority of each Party pursuant to Section 4.1(f)(ii)(A) or, as the case may be, Section 4.1(f)(ii)(B) also at the JCC level or decide to refer such dispute to the JSC for decision, and (B) any matter not falling within any of the categories of Section

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4.1(f)(ii)(A) or, as the case may be, Section 4.1(f)(ii)(B) shall be referred to the JSC for decision.

4.3 Alliance Managers. Each Party shall appoint an employee (or an employee of its Affiliate) to serve as an alliance manager ("Alliance Manager") with responsibility for overseeing that the Parties' activities are conducted in accordance with the Collaboration Agreements, and for being the primary point of contact between the Parties with respect to all such activities. The Alliance Managers to be appointed under this Agreement shall be identical to the ones to be appointed under the Co-Development and License Agreement. The Alliance Managers are responsible for driving the Collaboration progress and the resolution of issues between the Parties. The Alliance Managers may be members, but in any event may attend the meetings of the JSC, JCC or any other JSC subcommittee, and be responsible for communicating with and reporting to the JSC, JCC and any other JSC subcommittee on all relevant matters.

## ARTICLE V REPRESENTATIONS, WARRANTIES AND COVENANTS

5.1 Mutual Representations, Warranties and Covenants. Each Party hereby represents, warrants and covenants to the other Party, as of the Effective Date and, where expressly stated, at all times during the Term, as follows:

(a) Such Party: (i) is duly formed and in good standing under the laws of the jurisdiction of its formation, (ii) has the power and authority and the legal right to enter into this Agreement and perform its obligations hereunder, and (iii) has taken all necessary action on its part required to authorize the execution and delivery of this Agreement and the performance of its obligations hereunder;

(b) Upon execution, this Agreement will have been duly executed and delivered on behalf of such Party and constitutes a legal, valid and binding obligation of such Party and is enforceable against it in accordance with its terms;

(c) The execution and delivery of this Agreement and the performance of such Party's obligations hereunder: (i) do not conflict with or violate any requirement of Applicable Laws or any provision of the articles of incorporation, bylaws or limited partnership agreement of such Party; and (ii) do not conflict with, violate, or breach, or constitute a default or require any further consent under, any contractual obligation or court or administrative order by which such Party is bound;

(d) During the Term, to its knowledge, such Party will not, in the conduct of its activities under this Agreement, (i) employ or use any contractor or consultant that employs any individual or entity debarred by the FDA (or subject to a similar sanction of EMA), or (ii) employ any individual who or

entity that is the subject of an FDA debarment investigation or proceeding (or similar proceeding of EMA);

(e) During the Term, such Party shall perform its activities pursuant to this Agreement in compliance in all material respects with Applicable Laws;

(f) During the Term, neither Party shall grant any right or license to any Third Party relating to any of the Intellectual Property Rights it Controls which would

conflict with, or limit the scope of, any of the rights or licenses granted or to be granted to the other Party hereunder.

5.2 Additional Representations, Warranties and Covenants of uniQure. uniQure hereby represents, warrants and covenants as of the Effective Date, and, where expressly stated, at all times during the Term, that:

(a) During the Term, at the time the same are tendered to the carrier for delivery to Chiesi's customers, the Product sold to Chiesi pursuant to this Agreement (i) shall be Manufactured, stored, handled and released in compliance with all Applicable Laws, including GMPs; (ii) shall meet the applicable Specifications and (iii) shall not be adulterated or misbranded or otherwise defective within the meaning of any Applicable Laws;

(b) Schedule 1.63 attached hereto is a complete and correct list of all Patents that Cover the Product in the Territory and, subject to Section 9.1(b), are Controlled by uniQure as of the Effective Date;

(c) uniQure Controls all uniQure Intellectual Property and, subject to Section 9.1(b), has the full right, power and authority to grant to Chiesi the rights and licenses necessary to perform Chiesi's activities under this Agreement in the Territory;

(d) To uniQure's knowledge, the Commercialization of the Product in the Territory, as anticipated hereunder, does not infringe upon any Intellectual Property Rights of any Third Party;

(e) uniQure has not received any written allegation from a Third Party that any of the Patents issued on the Effective Date which is Controlled by UniQure and Covering the Product in the Territory is invalid or unenforceable and, to uniQure's knowledge, none of such Patents is infringed by any Third Party.

5.3 Additional Representations, Warranties and Covenants of Chiesi. Chiesi hereby represents, warrants and covenants as of the Effective Date, and, where expressly stated, at all times during the Term, that any and all Delivery Notification requirements set forth in Section 2.5(b) shall be fulfilled before any quantities of the Product are supplied to any of its customers.

5.4 No Other Representations or Warranties. EXCEPT AS OTHERWISE EXPRESSLY SET FORTH IN THIS AGREEMENT, NEITHER PARTY MAKES ANY REPRESENTATION OR EXTENDS ANY WARRANTY OF ANY KIND, EITHER EXPRESS OR IMPLIED, INCLUDING ANY WARRANTY THAT ANY PATENT RIGHTS ARE VALID OR ENFORCEABLE, AND EXPRESSLY DISCLAIMS ALL IMPLIED WARRANTIES OF MERCHANTABILITY, FITNESS FOR A PARTICULAR PURPOSE AND NON-INFRINGEMENT. WITHOUT LIMITING THE GENERALITY OF THE FOREGOING, EACH PARTY DISCLAIMS ANY WARRANTIES WITH REGARDS TO: (A) THE SAFETY, USEFULNESS FOR ANY PURPOSE OR NON-INFRINGEMENT OF ANY PRODUCT; OR (B) THE VALIDITY, ENFORCEABILITY OR NON-INFRINGEMENT OF ANY INTELLECTUAL PROPERTY RIGHTS IT PROVIDES OR LICENSES TO THE OTHER PARTY UNDER THIS AGREEMENT.

5.5 No Reliance by Third Parties. The representations and warranties of a Party set forth in this Article 5 are intended for the sole and exclusive benefit of the other Party hereto, and may not be relied upon by any Third Party.

## ARTICLE VI INDEMNIFICATION; INSURANCE

6.1 Indemnification by Chiesi. Chiesi shall indemnify, defend and hold harmless uniQure and its Affiliates, and its and their respective directors, officers, employees and agents, from and against any and all liabilities, damages, losses, costs and expenses, including the reasonable fees of attorneys and other professional Third Parties (collectively, "Losses"), arising out of or resulting from any and all Third Party suits, claims, actions, proceedings or demands ("Claims") to the extent based upon:

- (i) any breach of any representation, warranty or covenant made by, or any material obligation of, Chiesi under this Agreement;
- (ii) the gross negligence, recklessness or willful misconduct of Chiesi or its Affiliates and its or their respective directors, officers, employees and agents;
- (iii) any theory of product liability (including without limitation tort, warranty, or strict liability) that is applicable in the Territory with respect to the death, personal injury, or illness of any Person in the Territory, and arising directly from Chiesi's or its Affiliates' or Sub-distributors' Commercialization of the Product in the Territory;

provided that Chiesi shall not be obligated pursuant to this Section 6.1 if and to the extent uniQure is required to indemnify Chiesi under Section 6.2 below.

6.2 Indemnification by uniQure. uniQure shall indemnify, defend and hold harmless Chiesi and its Affiliates, and its and their respective directors, officers, employees and agents, from and against any and all Losses, arising out of or resulting from any and all Claims to the extent based upon:

- (i) any breach of any representation, warranty or covenant made by, or any material obligation of, uniQure under this Agreement;

(ii) the gross negligence, recklessness or willful misconduct of uniQure or its Affiliates and its or their respective directors, officers, employees and agents;

(iii) any theory of product liability (including without limitation tort, warranty, or strict liability) that is applicable in the Territory with respect to the death, personal injury, or illness of any Person in the Territory, and arising directly from uniQure's or its Affiliates' development, design, Manufacture, storage, release and handling of the Product;

(iv) Claims that the (i) Commercialization of the Product; or (ii) exercise of any rights or licenses granted to Chiesi and its Affiliates in accordance with this Agreement; violates or infringes upon the Intellectual Property Rights of any Third Party;

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provided that uniQure shall not be obligated pursuant to this Section 6.2 if and to the extent Chiesi is required to indemnify uniQure under Section 6.1 above.

### 6.3 Procedure.

(a) A Party entitled to indemnification under this Article VI (an "Indemnified Party") shall give prompt written notification to the Party from whom indemnification is sought (the "Indemnifying Party") of the commencement of any Claim for which indemnification may be sought or, if earlier, upon the assertion of any such Claim by a Third Party (it being understood and agreed, however, that the failure by an Indemnified Party to give notice of a Claim as provided in this Section 6.3(i) shall not relieve the Indemnifying Party of its indemnification obligation under this Agreement except and only to the extent that such Indemnifying Party is actually damaged as a result of such failure to give notice).

(b) Within [\*\*] days after delivery of such notification, the Indemnifying Party may, upon written notice thereof to the Indemnified Party, assume control of the defense of such Claim with counsel reasonably satisfactory to the Indemnified Party.

(c) If the Indemnifying Party does not assume control of such defense, the Indemnified Party shall control such defense and, without limiting the Indemnifying Party's indemnification obligations, the Indemnifying Party shall reimburse the Indemnified Party for all reasonable costs and expenses, including reasonable attorney's fees, incurred by the Indemnified Party in defending itself, within [\*\*] days after receipt of any invoice therefor from the Indemnified Party, such invoice to be issued no more often than quarterly.

(d) The Party not controlling such defense may participate therein at its own expense; provided that, if the Indemnifying Party assumes control of such defense and the Indemnified Party in good faith concludes, based on advice from counsel, that the Indemnifying Party and the Indemnified Party have conflicting interests with respect to such Claim, the Indemnifying Party shall be responsible for the reasonable fees and expenses of counsel to the Indemnified Party in connection with its participation in the defense action.

(e) The Party controlling such defense shall keep the other Party advised of the status of such Claim and the defense thereof and shall consider recommendations made by the other Party with respect thereto.

(f) The Indemnified Party shall not agree to any settlement of any Claim without the prior written consent of the Indemnifying Party, which shall not be unreasonably withheld, delayed or conditioned. The Indemnifying Party shall not, without the prior written consent of the Indemnified Party, agree to any settlement of such Claim, or consent to any judgment in respect thereof, that does not include a complete and unconditional release of the Indemnified Party from all liability with respect thereto, that imposes any liability or obligation on the Indemnified Party or that acknowledges fault by the Indemnified Party.

6.4 Limitation of Liability. EXCEPT WITH RESPECT TO ANY BREACH BY A PARTY OF ITS OBLIGATIONS UNDER ARTICLE X, EXCEPT AS

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PROVIDED FOR IN SECTION 2.6, EXCEPT FOR ANY DAMAGES ARISING FROM A PARTY'S WILLFUL MISCONDUCT AND EXCEPT TO THE EXTENT A PARTY MAY BE REQUIRED TO INDEMNIFY THE OTHER PARTY UNDER THIS ARTICLE VI WITH RESPECT TO THIRD PARTY CLAIMS, NEITHER PARTY SHALL BE LIABLE FOR ANY (AND EACH PARTY HEREBY DISCLAIMS ALL) SPECIAL, EXEMPLARY, CONSEQUENTIAL, PUNITIVE OR OTHER INDIRECT DAMAGES, INCLUDING LOST REVENUE AND LOST PROFITS, WHETHER BASED UPON WARRANTY, CONTRACT, TORT, STRICT LIABILITY OR OTHER LEGAL THEORY. EXCEPT WITH RESPECT TO ANY BREACH BY UNIQUE OF ITS OBLIGATIONS UNDER ARTICLE X, EXCEPT AS PROVIDED FOR IN SECTION 2.6, EXCEPT FOR ANY DAMAGES ARISING FROM UNIQUE'S WILLFUL MISCONDUCT AND EXCEPT TO THE EXTENT UNIQUE MAY BE REQUIRED TO INDEMNIFY CHIESI UNDER THIS ARTICLE VI WITH RESPECT TO THIRD PARTY CLAIMS, THE TOTAL LIABILITY OF UNIQUE, ITS AFFILIATES, AND THEIR RESPECTIVE OFFICERS, EMPLOYEES, AND AGENTS ARISING OUT OF OR IN RELATION TO THIS AGREEMENT, WHETHER BASED UPON WARRANTY, CONTRACT, TORT, STRICT LIABILITY OR OTHER LEGAL THEORY, SHALL FURTHER BE LIMITED TO AN AMOUNT OF EUR 10,000,000.00 (IN WORDS: TEN MILLION EURO) (REFLECTING THE AMOUNT PAYABLE UNDER UNIQUE'S INSURANCE PURSUANT TO SECTION 6.5) FOR THE CORRESPONDING DAMAGE EVENT. CHIESI SHALL REASONABLY COOPERATE WITH UNIQUE IN OBTAINING SUCH INSURANCE, AT UNIQUE'S COST.

6.5 Insurance. Each Party shall procure and maintain at its cost insurance, including product liability insurance, adequate to cover its obligations hereunder and which are consistent with normal business practices of comparable companies with respect to similar obligations and liabilities, at all times during the Term. In addition, uniQure shall further procure and maintain, at uniQure's cost, insurance adequate to cover its obligations under the in-license agreement with Xenon Pharmaceuticals Inc. dated 18 June 2001 and Chiesi shall reasonably cooperate with uniQure in obtaining such insurance. It is understood that such insurance shall not be construed to create any limit of either Party's obligations or liabilities with respect to its indemnification obligations hereunder. Each Party shall provide the other, upon request, with evidence of such insurance.

## ARTICLE VII INTELLECTUAL PROPERTY

7.1 Ownership. uniQure shall own or otherwise Control all right, title and interest in and to any uniQure Intellectual Property Rights.

7.2 Enforcement. uniQure shall have the exclusive right and the obligation, to institute infringement actions against any Third Parties (other than Sub-distributors) based on any Patents and other Intellectual Property Rights Covering the Product in the Territory. Chiesi shall execute all necessary and proper

documents and take such actions as shall be appropriate to allow uniQure to institute and prosecute such infringement actions and shall otherwise cooperate, at uniQure's expense, in the institution and prosecution of such actions. Upon reasonable request of Chiesi, uniQure (i) shall provide to Chiesi all reasonable information in connection with such infringement actions; (ii) shall allow a qualified representative of Chiesi to attend as an observer at relevant

negotiations and hearings, if and to the extent such attendance is both legally permitted and reasonably acceptable for uniQure and (iii) shall consider any measures suggested by Chiesi in connection with such infringement actions, it being understood that uniQure, without any obligation to state reasons for its decision, shall not be obliged to accept, fulfill or maintain such measures.

7.3 Right to Commercialize.

(a) During the Term and subject to the terms of this Agreement, in particular Section 9.1(b), uniQure hereby grants to Chiesi and its Affiliates a royalty-free right and license, with the right to grant sublicenses only to Sub-distributors, in the Territory to uniQure Intellectual Property Rights that are required to Commercialize the Product in the Territory under and in accordance with the terms of this Agreement. Such right and license shall be exclusive except in cases where, based on agreements between uniQure and Third Parties existing on the Effective Date, uniQure is not capable of granting exclusive but only non-exclusive licenses (e.g. because uniQure itself has only obtained non-exclusive rights and licenses from Third Party licensors).

(b) Chiesi shall not have the right to carry out any research or development with respect to the Product, or, subject to Sections 2.6, 9.3(b) and 9.3(c), to Manufacture the Product, or have the Product Manufactured by an Affiliate or Third Party.

7.4 Compliance with Third Party Agreement.

(a) The grants by uniQure under uniQure Intellectual Property Rights set forth in Section 7.3(a) include the sublicense of certain uniQure Intellectual Property Rights that are not owned by uniQure. Chiesi's rights and licenses under, or with respect to, uniQure Intellectual Property Rights, including any Patent prosecution or enforcement undertaken by the Parties pursuant to Section 7.2, are limited to the rights granted by Third Party licensors to uniQure under the respective in-license agreements between such Third Party licensors and uniQure ("Existing Third Party Licenses") and are subject to all applicable restrictions, limitations and obligations imposed on uniQure or its sub-licensees in such Existing Third Party Licenses, a copy of which agreements is attached hereto as Schedule 7.4. Chiesi shall comply, and cause its Affiliates and Sub-distributors to comply, with all such restrictions, limitations and obligations *mutatis mutandis*. To the extent there is a conflict between the terms of any Existing Third Party License and the rights granted to Chiesi hereunder, the terms of such Existing Third Party License shall control solely with respect to the Patents and know-how owned or controlled by the applicable Third Party licensor. Notwithstanding anything to the contrary in this Agreement, either Party may not exercise any of its rights under this Agreement (including any right to any cure period (including under Section 9.2(b)) or to delay performance of an obligation (including under Section 11.6)) in any manner that would result in any licensor having a right to terminate an Existing Third Party License, or that would cause the other Party to be in breach of any of its obligations under any Existing Third Party License.

(b) During the Term, uniQure shall comply with the Existing Third Party Licenses in effect which are then applicable to the activities under this Agreement with respect to the Product (and in particular shall not commit any breach that would entitle the Third Party licensor to terminate such an Existing Third Party License) and

shall not terminate any such Existing Third Party License without Chiesi's prior written consent. In addition, during the Term, uniQure shall promptly notify Chiesi of any written notice of breach or termination received by uniQure with respect to any such Existing Third Party License and, to the extent that uniQure does not cure such breach at least **[\*\*]** Business Days before the date on which the relevant licensor could terminate such Existing Third Party License due to such breach by uniQure, Chiesi shall have the right (to the extent consistent with such Existing Third Party License) to cure any such breach on uniQure's behalf and in such a case, Chiesi shall have the right to deduct (i) any and all arm's length payments made on behalf of uniQure for the above purpose, from the next due payments to be made hereunder plus (ii) interest on such payments calculated pursuant to Section 2.1(f) above.

(c) The license granted by uniQure in Section 7.3(a) with respect to the Patents licensed under the Existing Third Party Licenses are subject to rights reserved by the licensors and the US government as set forth in the Existing Third Party Licenses.

7.5 Additional Rights Acquired after Effective Date.

(a) During the Term, if either Party identifies the need for, or is otherwise offered, a license, covenant not to sue or similar rights to any Third Party Intellectual Property Rights that such Party in good faith believes is necessary or useful for the Commercialization of the Product in the Field in the Territory ("Additional Rights"), then such Party shall promptly notify the other Party and, in any event, prior to commencing negotiation or entering into an agreement with respect to such Additional Rights, and the Parties' rights to conduct such negotiations shall be subject to the remaining provisions of this Section 7.5. The Parties shall thereafter conduct good faith discussions regarding whether such Additional Rights are necessary or useful for the Commercialization of the Product in the Field in the Territory or whether they otherwise agree that such Additional Rights should be acquired.

(b) uniQure shall have the first right (but not the obligation) to license or otherwise acquire rights to any Additional Rights. If uniQure provides written notice to Chiesi that uniQure declines to exercise such first right, then Chiesi shall have the right (but not the obligation) to pursue acquiring rights to any given Additional Rights. The Party pursuing any given Additional Rights (the "Controlling Party") shall keep the other Party (the "Non-Controlling Party") reasonably informed regarding the status thereof and shall use Commercially Reasonable Efforts to obtain from the applicable Third Party licensor the right to sublicense such Additional Rights under the licenses granted to the Non-Controlling Party hereunder.

(c) If the Controlling Party acquires rights to any Additional Rights and has the right to grant a sublicense under such Additional Rights to the Non-Controlling Party, and the Non-Controlling Party wishes to include such Additional Rights in the licenses granted to the Non-Controlling Party hereunder, the Non-Controlling Party shall notify the Controlling Party of its desire to do so and the Controlling Party shall provide the Non-Controlling Party a summary of all material restrictions on the scope of the licenses granted under, and all material payment obligations that would be owed by the Non-Controlling Party with respect to, any Third Party agreement applicable to such Additional Rights. The Non-Controlling Party may, upon written notice to the Controlling Party and subject to Section 7.5(d), Section 7.5(e)

and Section 7.5(f), obtain a sublicense under such Additional Rights and include such Additional Rights under the licenses granted to the Non-Controlling Party hereunder.

(d) Following such notice from the Non-Controlling Party that it desires to include any given Additional Rights under the license granted to the Non-Controlling Party hereunder, (i) any such Additional Rights that do not carry financial or other obligations or restrictions shall be included automatically under the applicable license hereunder, and (ii) subject to Section 7.5(e) below, any such Additional Rights that carry financial or other obligations or restrictions shall be included under the applicable license hereunder only if the Non-Controlling Party agrees to share the costs of such Additional Rights (including any upfront payment or similar acquisition cost to access such Additional Rights) with the Controlling Party and to assume all other obligations to, and be subject to all restrictions imposed by, the Controlling Party's licensor to the extent arising from the grant to the Non-Controlling Party under such Additional Rights (including, to the extent access to such terms have been made available to the Non-Controlling Party in unredacted form, all other terms of the Additional Rights Agreement that apply to the licenses granted to the Non-Controlling Party hereunder).

(e) If the Parties are unable, after [\*\*] Business Days, to agree as to whether any given Additional Rights are in fact necessary or useful for the Commercialization of the Product in the Field in the Territory or if the Parties are unable to agree to the allocation of the costs (as specified above), then the Parties shall jointly engage an expert panel consisting of patent attorney(s) or expert(s) in the development, manufacturing or commercialization of products comparable to the Product in question and other CMC matters, as applicable, not regularly employed by either Party to resolve such dispute. The decision of such expert panel shall be binding on the Parties as to such dispute.

(f) Nothing in this Section 7.5 shall restrict either Party, at such Party's sole cost and expense, from licensing or otherwise acquiring any additional rights that are not necessary or useful for the Commercialization of the Product in the Field in the Territory.

## ARTICLE VIII COMMERCIALIZATION

### 8.1 Commercialization.

(a) Chiesi shall have the sole right and responsibility, at its expense, to Commercialize the Product in the Field in the Territory, including for booking all sales of the Product throughout the Territory. At all times during the Term Chiesi shall in no event use less than Commercially Reasonable Efforts to Commercialize the Product, including compliance with marketing plan and budget, allocation of Minimum FTEs and setting of a Target Price (as defined in Schedule 8.1(a)) in Germany, as further described in Schedule 8.1(a). Notwithstanding the foregoing, Chiesi shall at least use Commercially Reasonable Efforts to achieve the First Commercial Sale of the Product in the Territory in the second half of 2013, provided that uniQure shall ensure availability of sufficient quantities of Product for supply to Chiesi's customers for such purpose, prior to the First Commercial Sale. In the event Chiesi fails to meet (i) the allocation of Minimum FTEs or (ii) the timelines for submission of each relevant dossier for obtaining the Price and Reimbursement Approval for the Product, as further described in Schedule 8.1(a), and

such failure is not caused by a Force Majeure Event or uniQure's Failure to Supply, and Chiesi fails to cure such failure within [\*\*] months after receiving written notice of such failure, uniQure shall have the right to terminate this Agreement in its entirety in the event of a failure as described in sub-paragraph (i) above or, at the sole discretion of uniQure, with respect to the particular countries to which such failure relates in the event of a failure as described in sub-paragraph (ii) above.

(b) In order to prevent a substantial delay in achieving the First Commercial Sale of the Product in the Territory, certain commercial and development activities have been committed to by uniQure prior to the Effective Date (the "Approved Activities") attached in Schedule 8.1(b). Such Approved Activities shall be reimbursed by Chiesi at uniQure's actual cost.

(c) Chiesi shall have the sole authority to determine the resale price of the Product in the Field in the Territory.

### 8.2 Exclusivity.

(a) During the Term, Chiesi shall not actively market, advertise for, canvas for or seek orders for the Product outside the Field or Territory or establish any branch, subsidiary, or depot for the supply of the Product outside the Field or Territory. The Parties shall inform each other promptly in case of any Commercialization activities of Third Parties in the Field in the Territory, or of any Commercialization activities of Chiesi or any of its Affiliates outside the Field or Territory to agree — within the limits of applicable competition laws — on any appropriate measures to be taken.

(b) During the Term, uniQure shall not offer for sale, sell, license or otherwise Commercialize the Product in the Territory other than in compliance with the terms of this Agreement. uniQure shall be free, however, at any time during the Term, to Commercialize, directly or indirectly, the Product outside the Territory.

(c) To the fullest extent consistent with any Applicable Laws, each Party shall, and shall procure that any of its Affiliates will, not directly or indirectly, itself or through or with or on behalf of any Third Party, develop, Manufacture or Commercialize in the Territory any Gene Therapy based product characterized to treat lipoprotein lipase deficiency, other than the Product in accordance with this Agreement. From time to time during the Term, the Parties may negotiate exceptions for Persons which will become Affiliates of a Party due to an acquisition of or by a Party or its Affiliates.

## ARTICLE IX TERM AND TERMINATION

### 9.1 Term.

(a) *General.* This Agreement shall become effective as of the Effective Date and shall remain in force, on a country-by-country basis, for the longer of (i) twelve (12) years from the First Commercial Sale of the Product in the relevant country of the Territory; (ii) expiry of any regulatory exclusivity granted by any Marketing Authorization or any other Regulatory Approval in the relevant country of the Territory; or (iii) expiry of the last Valid Claim Covering the Product in the relevant

country of the Territory. Unless terminated by a Party with three (3) months written notice to the other Party to the end of the above initial or any subsequent term, this Agreement shall automatically be renewed for successive five (5) year terms (the initial and each subsequent term, the “Term”).

(b) *Condition Precedent.* This Agreement, except for the obligation to submit the first Firm Order in accordance with Section 2.4(b), and any ancillary agreement concluded between the Parties in connection herewith, including the Quality Agreement and the SDEA, and the Co-Development and License Agreement and the agreement regarding the equity investment of Chiesi in uniQure concluded on the date hereof, shall become effective once the Parties have received consent from or, as the case may be, entered into separate agreements with, the respective Third Party licensors to the subcontracting of the rights and licenses licensed by uniQure as licensee under the Existing Third Party Licenses listed in Schedule 9.1 to Chiesi. uniQure and, to the extent applicable, Chiesi, shall use Commercially Reasonable Efforts to obtain such consent or, as the case may be, enter into such agreements, on or prior to [\*\*]. If, despite the Parties’ Commercially Reasonable Efforts, such consent has not been obtained from or, as the case may be, such agreements have not been entered into with, all such Third Party licensors by the end of [\*\*], this Agreement and all other agreements that are subject to the condition precedent pursuant to sentence 1 shall be deemed null and void as of the Effective Date and the first Firm Order submitted by Chiesi in accordance with Section 2.4(b) shall be deemed withdrawn, unless, prior to the end of such period, following a corresponding request of either Party, the Parties mutually agree in writing on an extension of such period. The Parties agree that (i) costs and expenses incurred in connection with the preparation and execution of this Agreement as well as obtaining of the aforementioned consent or, as the case may be, enter into the aforementioned agreements, shall not be reimbursed, provided, however, that uniQure shall pay back to Chiesi any payments received in connection with this Agreement on or prior to [\*\*] (or such extended period mutually agreed between the Parties in accordance with the foregoing), and (ii) Sections 10.1, 10.2 and 10.6 shall apply *mutatis mutandis*.

9.2 Termination. Without prejudice to any other termination rights set forth herein, the Parties shall have the following termination rights:

(a) *Mutual Agreement.* The Parties may terminate this Agreement at any time during the Term upon mutual agreement in writing.

(b) *Material Breach.* Either Party may immediately terminate this Agreement in writing if the other Party materially breaches this Agreement and fails to cure such breach within [\*\*] days after receiving written notice of the breach.

(c) *Insolvency.* Either Party may immediately terminate this Agreement in writing if the other Party ceases to carry on business, is unable to pay its debts when they fall due, is declared bankrupt, or an order is made or a resolution passed for the winding up of that other Party or the appointment of an administrator, receiver, liquidator, or manager of that other Party.

(d) *IP Challenge.* Either Party may immediately terminate this Agreement in writing if the other Party or any of its Affiliates or, as the case may be, Sub-distributors challenges the validity of any trademark as set forth in Section 2.2(a) or if

Chiesi or any of its Affiliates or Sub-distributors challenges the validity, enforceability, patentability or scope of any Valid Claim included in any Patents.

9.3 Effects of Expiration/Termination.

(a) Upon termination of this Agreement by uniQure pursuant to Sections 8.1(a), 9.2(b), 9.2(c) or 9.2(d):

(i) Chiesi shall purchase from uniQure any quantity of Product which has been included in a Confirmed Firm Order through the effective date of termination, unless otherwise elected by UniQure pursuant to Section 9.3(a)(ii) below;

(ii) (A) all rights, privileges and licenses granted hereunder to Chiesi shall remain in full force and effect until all quantities of Product ordered and delivered hereunder, at the election of uniQure, (y) have been sold by Chiesi, or (z) have been redeemed by uniQure from Chiesi at the Purchase Price originally charged to Chiesi except for such portion of Product as is needed to fill orders then held by Chiesi; and (B) Chiesi shall thereafter not make any use whatsoever of any such rights, privileges and licenses and transfer to uniQure any Marketing Authorization then held by Chiesi or its Sub-distributor, unless required by Applicable Laws or expressly set forth otherwise in this Agreement;

(iii) save as required under the Quality Agreement or the SDEA, at any time upon written request of the disclosing Party, unless expressly set forth otherwise in this Agreement, the receiving Party shall cease use of and return or at the disclosing Party’s request destroy all Confidential Information of the disclosing Party and all copies thereof except for a single copy of such Confidential Information that may be retained confidentially for legal purposes only;

(iv) all rights, privileges and licenses granted hereunder to uniQure regarding any alternative Trademark identified by Chiesi and any other trademarks, logos or service marks of Chiesi shall become fully paid-up, irrevocable and perpetual.

(b) Upon termination of this Agreement by Chiesi pursuant to Sections 9.2(b), 9.2(c) or 9.2(d):

(i) all rights, privileges and licenses granted hereunder to Chiesi regarding the uniQure Intellectual Property Rights, including the rights granted under Section 2.2(a), shall become fully paid-up, irrevocable and perpetual;

(ii) all rights, privileges and licenses granted hereunder to uniQure shall terminate and uniQure shall not make any use whatsoever of any alternative Trademark identified by Chiesi and any other trademarks, logos or service marks of Chiesi, unless required by Applicable Laws or expressly set forth otherwise in this Agreement;

(iii) uniQure shall furnish Chiesi with reasonable cooperation, and continue to supply Chiesi’s requirements of Product for the [\*\*] month period following notice of termination in accordance with the terms and conditions of this Agreement, provided however, that the Purchase Price for the individual Product ordered



after the effective date of termination shall be the Fully Loaded Cost of Goods plus [\*\*] percent ([\*\*]%) markup for each patient dose sold of such particular Product. No later than [\*\*] months prior to the expiration of such [\*\*] month period the Parties shall enter into good faith negotiations regarding the supply of Chiesi's requirements of Product after expiration of such [\*\*] month period, taking into account a fair adjustment of the transfer price pursuant to Section 2.3(b) for the Product to be supplied to Chiesi after such expiration;

(iv) save as required under the Quality Agreement or the SDEA, at any time upon written request of the disclosing Party, unless expressly set forth otherwise in this Agreement, the receiving Party shall cease use of and return or at the disclosing Party's request destroy all Confidential Information of the disclosing Party and all copies thereof except for a single copy of such Confidential Information that may be retained confidentially for legal purposes only.

(c) Upon expiration of the Term with respect to this Agreement by a Party exercising its termination right pursuant to Section 9.1(a) or mutual termination pursuant to Section 9.2(a) (unless otherwise agreed between the Parties in such mutual termination agreement):

(i) all rights, privileges and licenses granted hereunder to Chiesi shall become fully paid-up, irrevocable and perpetual;

(ii) all rights, privileges and licenses granted hereunder to uniQure shall become fully paid-up, irrevocable and perpetual;

(iii) Chiesi shall purchase from uniQure any quantity of Product which has been included in a Confirmed Firm Order through the effective date of expiration;

(iv) save as required under the Quality Agreement or the SDEA, at any time upon written request of the disclosing Party, unless expressly set forth otherwise in this Agreement, the receiving Party shall cease use of and return or at the disclosing Party's request destroy all Confidential Information of the disclosing Party and all copies thereof except for a single copy of such Confidential Information that may be retained confidentially for legal purposes only.

Upon expiration of the Term with respect to this Agreement by uniQure exercising its termination right pursuant to Section 9.1(a), uniQure shall continue to supply Chiesi's requirements of Product for the [\*\*] month period following notice of termination in accordance with the terms and conditions of this Agreement, provided however, that the Purchase Price for the individual Product ordered after the effective date of termination shall be the Fully Loaded Cost of Goods plus [\*\*] percent ([\*\*]%) markup for each patient dose sold of such particular Product. No later than [\*\*] months prior to the expiration of such [\*\*] month period the Parties shall enter into good faith negotiations regarding the supply of Chiesi's requirements of Product after expiration of such [\*\*] month period, taking into account a fair adjustment of the transfer price pursuant to Section 2.3(b) for the Product to be supplied to Chiesi after such expiration.

(d) *Accrued Rights; Surviving Provisions.*

(i) Notwithstanding the giving of any notice of termination pursuant to this Article 9, each Party shall continue to fulfill such Party's obligations under this Agreement at all times until the effective date of any such termination.

(ii) Termination or expiration of this Agreement for any reason shall be without prejudice to any rights which shall have accrued to the benefit of either Party prior to such termination or expiration.

(iii) Without prejudice to Section 11.7, to the extent legally permitted, any compensation claims by Chiesi resulting from a direct or analogous application of Article 17 of Council Directive 86/653/EEC, as amended, as transposed into the national laws of the EU Member States for undertaking the Commercialization of the Product in the Territory are expressly excluded and hereby expressly waived by Chiesi.

(iv) All those provisions which by their scope and nature extend beyond the Term, including Article I, Section 2.1(d) to (h), Article VI, Section 7.1, Section 9.3, Sections 10.1, 10.2 and 10.6, and Article XI, shall survive any expiration or termination of this Agreement, and remain in full force and effect.

## ARTICLE X CONFIDENTIALITY

10.1 Confidential Information. All Confidential Information disclosed by a Party or any of its Affiliates to the other Party or any of its Affiliates before or during the Term shall not be used by the receiving Party or any of its Affiliates except in connection with the activities contemplated by this Agreement, shall be maintained in confidence by the receiving Party and its Affiliates, and shall not otherwise be disclosed by the receiving Party or its Affiliates to any Third Party (except as set forth in the remainder of this Article X), without the prior written consent of the disclosing Party, except to the extent that the Confidential Information:

(a) was known or used by the receiving Party or any of its Affiliates prior to its date of disclosure by the disclosing Party;

(b) either before or after the date of the disclosure to the receiving Party hereunder or under the Confidentiality Agreement is lawfully disclosed to the receiving Party or any of its Affiliates by a Third Party rightfully in possession of and with the right to disclose such Confidential Information other than under an obligation of confidentiality;

(c) either before or after the date of the disclosure to the receiving Party hereunder or under the Confidentiality Agreement becomes generally known to the public through no fault or omission on the part of the receiving Party or its Affiliates;

(d) is independently developed by or for the receiving Party or any of its Affiliates without reference to or reliance upon any of the other Party's Confidential Information; or

(e) is required to be disclosed by the receiving Party or its Affiliates to comply with Applicable Laws, which may include the rules of Euronext, of the US

Securities and Exchange Commission, or of any other stock exchange, or to defend or prosecute litigation or arbitration or to comply with legal process; provided that, the receiving Party provides prior written notice of such disclosure to the disclosing Party (to the extent feasible) and only discloses Confidential Information of the other Party to the extent necessary for such legal compliance or litigation purpose; and provided, further, that (i) the receiving Party shall use, or shall cause its Affiliates, as the case may be, to use, reasonable efforts to obtain confidential treatment, or the equivalent, from Euronext, the US Securities and Exchange Commission, or other securities trading institution of any financial information or other information of a competitive or confidential nature, and shall include in such confidentiality request such provisions of this Agreement as may be reasonably requested by the disclosing Party, and (ii) such information shall otherwise remain Confidential Information (subject to the exceptions in this Section 10.1).

Notwithstanding the foregoing, paragraphs (a), (b) and (d) shall not alter the requirement to keep the terms and conditions of this Agreement confidential, as set forth herein, subject to the remainder of this Article X.

10.2 Employee, Director, Consultant and Advisor Obligations. Chiesi and uniQure each agrees that it and its Affiliates shall provide Confidential Information received from the other Party only to the receiving Party's employees, directors, consultants, agents and advisors, and to the employees, directors, consultants, agents and advisors of the receiving Party's Affiliates, who have a need to know such Confidential Information to assist the receiving Party in fulfilling its obligations under this Agreement and who are bound by obligations of confidentiality and non-use that are at least as restrictive as those set forth in this Agreement. Each Party shall remain responsible for any failure by any of its or its Affiliates' employees, directors, consultants, agents and advisors to treat such Confidential Information as required under this Article X.

### 10.3 Publicity.

(a) Following execution of this Agreement, the Parties shall jointly or separately issue a press release, in a text to be agreed upon between the Parties in advance, announcing the execution of this Agreement and the Co-Development and License Agreement.

(b) Each Party shall only issue press releases (other than the press release pursuant to paragraph (a) above) or make other public disclosures regarding this Agreement or the Parties' activities under this Agreement (each such press release or public disclosure, a "Subject Disclosure"):

(i) that have been approved in writing in advance by the other Party (such approval not to be unreasonably withheld, conditioned or delayed), including Subject Disclosures that describe one or more of the following:

- (A) the filing for or receipt of Marketing Authorization with respect to the Product in the Territory;
- (B) the receipt of Price and Reimbursement Approval for the Product in the Territory;

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- (C) the receipt of any regulatory exclusivity for the Product in the Territory;
- (D) the achievement of any commercial milestone pursuant to Section 2.1(c)(ii); or
- (E) the first Party's presence or participation at scientific, financial or investor forums;

(ii) subject to Section 10.3(c), if advised by counsel to issue such Subject Disclosure in order to comply with Applicable Laws, which may include the disclosure rules of the US Securities and Exchange Commission or a similar regulatory agency in a country in the Territory or of Euronext or any other stock exchange of other securities trading institution; or

(iii) subject to Section 10.3(c), if the contents of such Subject Disclosure have previously been made public other than through a breach of this Article X by a Party.

(c) Unless not feasible under the circumstances because of the need to comply with Applicable Laws or stock exchange rules, the Party making a Subject Disclosure shall provide the other Party with a draft Subject Disclosure at least [\*\*] Business Days prior to its intended publication for the other Party's review. Such other Party may provide the first Party with suggested modifications to the draft Subject Disclosure. The first Party shall consider in good faith the other Party's timely provided suggestions in issuing such Subject Disclosure.

(d) For clarity, nothing in this Agreement shall restrict (i) each Party from issuing press releases or making other public disclosures regarding such Party's development, manufacturing or commercialization activities with respect to any product other than the Product, or (ii) uniQure from issuing press releases or making other public disclosures regarding uniQure's development, manufacturing or commercialization activities with respect to the Product outside the Field or Territory.

10.4 Other Disclosures. Notwithstanding anything in this Agreement to the contrary, each Party shall have the right to disclose the other Party's Confidential Information (including the terms of this Agreement) (as applicable):

(a) to such Party's then-current or potential investors, lenders, acquirers, investment bankers, and other Third Parties in connection with financing, partnering (to the extent consistent with this Agreement) and acquisition activities, solely on a need-to-know basis and under obligations of confidentiality and non-use that are at least as restrictive as those set forth in this Article X;

(b) as required by the existing license agreements between uniQure and its Third Party licensors;

(c) to enforce Patents, Trademarks and other Intellectual Property Rights in accordance with Sections 2.2(a) and 7.2; or

(d) to such Party's then-current or potential collaborators, and Third Party contractors (including contract manufacturers and Sub-distributors) for purposes of

engaging in the Manufacture or Commercialization of the Product as contemplated hereunder, solely on a need-to-know basis and under obligations of confidentiality and non-use that are at least as restrictive as those set forth in this Article X.

#### 10.5 Publications.

(a) Notwithstanding Section 10.3 and Section 10.4, a Party (the “Publishing Party”) which is, or whose Affiliates is, seeking to publish or publicly present scientific or technical data, results or other information with respect to the Product shall provide the other Party and the JCC with a copy of any proposed publication or presentation at least [\*\*] days (or at least [\*\*] days in the case of abstracts or oral public presentations) prior to submission for publication or presentation so as to provide such other Party with an opportunity to recommend any changes it reasonably believes are necessary to continue to maintain such other Party’s Confidential Information in accordance with the requirements of this Agreement or to not jeopardize the patentability of any results or data.

(b) If the non-Publishing Party notifies the Publishing Party that such publication or presentation, in the non-Publishing Party’s reasonable judgment, (i) discloses an invention for which the non-Publishing Party desires to seek patent protection, or (ii) contains any Confidential Information of the non-Publishing Party, or could be expected to have an adverse effect on the commercial value of any Confidential Information disclosed by the non-Publishing Party to the Publishing Party, the Publishing Party shall delete such Confidential Information from the proposed publication or presentation and shall further delay such publication or presentation for a period reasonably sufficient to permit the timely preparation and filing of a patent application(s) on any invention disclosed in such publication or presentation (but no more than [\*\*] days from the date of the non-Publishing Party’s notice thereof).

10.6 Term. All obligations of confidentiality imposed under this Article X shall expire [\*\*] years following termination or expiration of this Agreement, except to the extent any Existing Third Party License between uniQure and its Third Party licensors extends such obligations; provided, however, that the receiving Party shall maintain the confidentiality of any of the other Party’s trade secrets indefinitely until such trade secret is no longer a trade secret.

### ARTICLE XI MISCELLANEOUS

11.1 Entire Agreement, Amendments. This Agreement and the attachments hereto contain the entire understanding and agreement of the Parties with respect to the subject matter hereof and cancel and supersede any and all prior negotiations, correspondence, understandings and agreements between the Parties, whether oral or written, regarding such subject matter, including the Memorandum of Understanding dated 21 December 2012 and the Confidentiality Agreement, but expressly excluding the Co-Development and License Agreement. Except for the rights expressly conferred on the JSC or JCC, this Agreement cannot be modified except by a written document bearing the signatures of both Parties. The same applies to any waiver of this written form requirement.

11.2 Assignments. Except as expressly provided herein, neither this Agreement nor any rights and obligations hereunder shall be assignable by a Party without the prior written consent of the other Party; provided, however, that a Party may assign this Agreement to any Affiliate or to any successor in interest by way of merger, acquisition or sale of all or substantially all of its assets to which this Agreement relates, provided that such successor agrees in writing to be bound by the terms of this Agreement as if it were the assigning Party. This Agreement shall be binding upon the successors and permitted assigns of the Parties. Any assignment not in accordance with this Section 11.2 shall be void.

11.3 Severability. Should any provision of this Agreement be or become invalid, ineffective or unenforceable as a whole or in part, the validity, effectiveness and enforceability of the remaining provisions shall not be affected thereby. Any such invalid, ineffective or unenforceable provision shall be deemed replaced by such valid, effective and enforceable provision as comes closest to the economic intent and the purpose of such invalid, ineffective or unenforceable provision. The aforesaid shall apply mutatis mutandis to any gap in this Agreement.

11.4 Notices. Other than as expressly specified in this Agreement, all notices and consents required to be provided hereunder shall be in writing and provided by hand, by recorded delivery mail (return receipt requested), by facsimile, or by recognized overnight courier service to the other Party at its address or facsimile number shown below or such other address or facsimile number notified by such other Party from time to time.

If to uniQure, addressed to:

uniQure Biopharma B.V.  
P.O. Box 22506  
1100 DA Amsterdam  
The Netherlands  
Attention: CEO  
Fax: +31 20 566 9272

If to Chiesi, addressed to:

Chiesi Farmaceutici S.p.A.  
Via Palermo, 26/A  
43122 Parma  
Italy  
Attention: CEO  
Copy to: Corporate Development, Head and General Counsel  
Fax: +39 0521 774468

11.5 Waiver. Any failure of either Party to enforce any provision hereof shall not constitute a waiver by that Party of its right subsequently to enforce the same or any other provision hereof. The waiver of any provision of this Agreement shall only be effective if in writing signed by the Party claimed to have waived such provision.

11.6 **Force Majeure.** Neither Party shall lose any rights hereunder or be liable to the other Party for damages or losses on account of failure of performance by the defaulting Party if the failure is occasioned by war, civil insurrection, strike, fire, Act of God, earthquake, tempest, flood, epidemic, blackout, lockout, embargo, governmental acts or orders or restrictions, delays in delivery and non-supply by exclusive suppliers, where such delay or non-supply occurs as a result of such Force Majeure, or any other reason where failure to perform is beyond the reasonable control of such Party (each a “**Force Majeure Event**”) and such failure to perform is not caused by the negligence, intentional conduct or misconduct of the non-performing Party and such Party has exerted all reasonable efforts to avoid or remedy such Force Majeure Event; provided, however, that in no event shall a Party be required to settle any labor dispute or disturbance.

11.7 **Independence.** The relationship between the Parties is that of independent contractors and neither Party shall have the power to bind or obligate the other Party in any manner. It is further understood and agreed that neither Party nor its Affiliates, nor its and their respective directors, officers and employees, shall be deemed an agent or employee of the other Party or its Affiliates.

11.8 **Third Party Beneficiaries.** All rights, benefits and remedies under this Agreement are solely intended for the benefit of uniQure and Chiesi. No Third Party shall have any rights whatsoever to (i) enforce any obligation contained in this Agreement; (ii) seek a benefit or remedy for any breach of this Agreement; or (iii) take any other action relating to this Agreement under any legal theory, including but not limited to, actions in contract, tort (including negligence, gross negligence and strict liability), or as a defense, setoff or counterclaim to any action or claim brought or made by the Parties.

11.9 **Governing Law, Dispute Resolution.** The validity and interpretation of this Agreement shall be governed by the laws of England without regard to its conflicts of laws principles and to the express exclusion of the United Nations Conventions on Contracts for the International Sale of Goods (CISG). Any dispute arising under, out of or relating to this Agreement shall be referred to and finally determined under the Rules of Arbitration of the International Chamber of Commerce by three (3) arbitrators appointed in accordance with the said Rules. The place of arbitration shall be London, United Kingdom. The language to be used in said proceedings shall be English.

11.10 **Costs.** Except as expressly provided in this Agreement or as separately agreed upon in writing between the Parties, each Party shall bear its own costs incurred in connection with the implementation of the obligations under this Agreement.

11.11 **Construction.** Each Party agrees that this Agreement shall be interpreted without regard to any presumption or rule requiring construction against the Party causing this Agreement to be drafted.

11.12 **Language.** This Agreement shall be written and executed in, and all other communications under or in connection with this Agreement shall be in, the English language. Any translation into any other language shall not be an official version thereof, and in the event of any conflict in interpretation between the English version and such translation, the English version shall control.

11.13 **Counterparts.** This Agreement may be executed simultaneously in any number of counterparts, any one of which need not contain the signature of more than one

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Party but all such counterparts taken together shall constitute one and the same agreement. A pdf file of this Agreement contained in an email, including the signed signature pages hereto, will be deemed to be an original.

*[Signature Page Follows]*

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IN WITNESS WHEREOF, the Parties have executed this Commercialization Agreement as of the Effective Date.

**UNIQURE BIOPHARMA B.V.**

**UNIQURE BIOPHARMA B.V.**

By: /s/ Piers Morgan  
Name: Mr. Piers Morgan  
Title: Chief Financial Officer

By: /s/ Hans Preusting  
Name: Mr. Hans Preusting  
Title: Business Development, Vice President

**CHIESI FARMACEUTICA S.p.A.**

**CHIESI FARMACEUTICI S.p.A.**

By: /s/ Alberto Chiesi  
Name: Mr. Alberto Chiesi  
Title: President

By: /s/ Ugo Di Francesco  
Name: Mr. Ugo Di Francesco  
Title: CEO

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**SCHEDULE 1.9**  
**Certificate of Analysis**

CONTROLLED DOCUMENT — CONFIDENTIAL

**Certificate of Analysis**

**uniQure**

Page 1 of 1

Remarks:

## Conclusion

Authorized signature:

Name: \_\_\_\_\_

Date: \_\_\_\_\_

Visiting address  
Meibergdreef 61  
1105 BA Amsterdam  
The Netherlands

Postal address  
P.O. Box 22506  
1100 DA Amsterdam  
The Netherlands

tel +31 (0)20 566 7394  
fax +31 (0)20 566 9272  
info@uniquire.com

**SCHEDULE 1.10**  
**Certificate of Compliance**

Document No.: QA-SOP-XXX-0036-EX  
Revision No.:  
Page No.: 1 of 1  
Effective date:

## Preparation of Certificates — Exhibit X: Certificate of Release



Product name:  
Quantity:  
Batch number:

Date manufactured:  
Expiry/retest date:  
Storage conditions:

Manufacturer: UniQure  
Production Site: Meibergdreef 61, 1105 BA Amsterdam, The Netherlands

**Release tests:**

o: All test results are within approved specifications.

**Certification statement:**

uniQure is certified by the Dutch Health Authorities (Ministerie van VWS), per manufacturing license number 108990F, to manufacture biological products (gene therapies).

I hereby certify that this batch has been manufactured at the above-stated site in full compliance with the EU GMP requirements, and meets the authorized quality specifications registered in uniQures’ quality systems. The batch manufacturing and analytical records were reviewed and are found to be in compliance with GMP.

Name:  
Position: Qualified Person  
Signature:

Date:

SCHEDULE 1.63  
Patents

UniQure Patent Portfolio: GLYBERA

UniQure Ref.	Country	Owners	Official No.	Case Status	Filing Date	Date Registration

Confidential Materials omitted and filed separately with the Securities and Exchange Commission. A total of four pages were omitted. [\*\*]

In-licensed patent portfolio: GLYBERA

Licensor	Owners	Official No.	Case Status	Filing Date	Date Registration
[**]					
[**]	[**]	[**]	[**]	[**]	[**]
[**]					
[**]	[**]	[**]	[**]	[**]	[**]
			[**]		
		[**]	[**]	[**]	[**]
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[**]	[**]	[**]	[**]	[**]	[**]
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[**]	[**]	[**]	[**]	[**]	[**]
[**]					
[**]	[**]	[**]	[**]	[**]	[**]
			[**]		

Note:

[\*\*]

SCHEDULE 1.66  
Product

Glybera: AAV1\_LPLS447X

Marketing Authorization Numbers: EU/1/12/791/001

SCHEDULE 1.78  
Initial Specifications

Before QC testing the samples taken from the drug product batch are stored at the same conditions and subjected to one freeze thaw cycle.

The proposed release and shelf life specifications for Glybera drug product are shown in Table 1 below:


**Table 1: Release and shelf life specifications for Glybera**

[illegible]

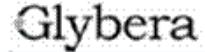
	[**]
[**]	[**]
[**]	[**]
[**]	

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## uniQure's Trademark Portfolio: GLYBERA - uniQure

Catchword	Type	Country	Classes	Appl.No.	Appl.date	Reg.No.	Rcg.datc	Rcn.date	Applicant	Status
										
G device	Logotype	EU	05, 44	8640609	10/26/2009.	8640609	5/10/2010.	10/26/2019.	uniQure IP B.V.	Registered
GLYBERA	Wordmark	EU	05, 44	5901269	5/1/2007.	5901269	5/14/2009.	5/1/2017.	uniQure IP B.V.	Registered
GLYBERA	Wordmark	TR	05	2007026778	17.05.2007	2007026778	17.05.2007	17.05.2017	uniQure IP B.V.	Registered
GLYBERA	Wordmark	RU	05	2008707340	13.03.2008	377215	20.04.2009	13.03.2018	Amsterdam Molecular Therapeutics (AMT) Holding N.V.	Registered
GLYBERA	Wordmark	CH	05	551392007	14.05.2007	562178	11.09.2007	14.05.2017	Amsterdam Molecular Therapeutics (AMT) Holding N.V.	Registered
GLYBERA	Wordmark	IS	05	14642007	14.05.2007	8122007	04.07.2007	04.07.2017	Amsterdam Molecular Therapeutics (AMT) Holding N.V.	Registered
GLYBERA	Wordmark	NO	05	200705606	15.05.2007	241553	19.10.2007	19.10.2017	Amsterdam Molecular Therapeutics (AMT) Holding N.V.	Registered
GLYBERA	Wordmark	DZ	05	72791	24.10.2007				Amsterdam Molecular Therapeutics (AMT) Holding N.V.	Pending
GLYBERA	Wordmark	EG	05	208229	22.10.2007				Amsterdam Molecular	Pending

GLYBERA	Wordmark	MA	05	113550	23.10.2007	113550	23.10.2007	23.10.2017	Therapeutics (AMT) Holding N.V.	Registered
GLYBERA	Wordmark	TN	05	EE072667	24.10.2007	EE72667	19.05.2009	24.10.2017	Amsterdam Molecular Therapeutics (AMT) Holding N.V.	Registered

	Logotype	EU	05, 44	8640641	10/26/2009.	8640641	5/10/2010.	10/26/2019.	uniQure IP B.V.	Registered
GLYBERA device UNIQUIRE	Wordmark	EU	01, 05, 42, 44	10431005	11/21/2011.		4/25/2012	11/21/2021.	uniQure IP B.V.	Registered

SCHEDULE 2.2(b)  
uniQure Trademark Guidelines

uniQure

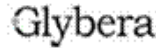
TRADEMARK GUIDELINES


The trademarks of uniQureBiopharmaB.V. and its subsidiary uniQure IP B.V. -hereinafter “uniQure” or “Company” - are valuable and important intellectual property assets of the Company. It is crucial that you protect the value of our trademarks by using them properly. These guidelines, which are updated from time to time, set out our policies for your use of such assets.

If you are a licensee of uniQure trademarks, your license agreement will specify the trademarks that you are authorized to use and may provide additional special trademark usage guidelines. You may NOT use our trademarks in a manner that incorrectly suggests that uniQure sponsors or endorses or is otherwise associated with your activities, products, and services, except as set forth in your license agreement with us.

Registered trademarks:

UniQure is owner of the following trademark registrations in the European Union:

Trademark	Type	Country	goods/services	Reg. No.	Reg. Date
GLYBERA	Word	EU	<b>Class 5:</b> Pharmaceutical products; biological preparations for use in medical and clinical gene therapy and cell therapy; clinical medical reagents for use in gene therapy; gene diagnosis, and gene testing; pharmaceutical preparations, vaccines for use in gene therapy; gene therapy and prophylaxis products; all the aforementioned goods exclusively in the treatment of metabolic disorders including such disorders which are single gene disorders and disorders which are a result of one more mutations within the lipoprotein lipase gene	5901269	5/14/2009
	word and device	EU	<b>Class 44:</b> Gene delivery, gene transfer, gene regulation and gene modulation for the treatment of metabolic disorders, including such disorders which are single gene disorders, and disorders which are a result of one more mutations within in the lipoprotein lipase gene, ocular disorders <b>Class 5:</b> Pharmaceutical products; biological preparations for use in medical and clinical gene therapy and cell therapy; clinical medical	8640641	5/10/2010

Trademark	Type	Country	goods/services	Reg. No.	Reg. Date
			reagents for use in gene therapy, gene diagnosis, and gene testing; pharmaceutical preparations, vaccines for use in gene therapy; gene therapy and prophylaxis products, all the aforementioned goods exclusively in the treatment of metabolic disorders including such disorders which are single gene disorders and disorders which are a result of one more mutations within the lipoprotein lipase gene		
	device	EU	<b>Class 44:</b> Gene delivery, gene transfer, gene regulation and gene modulation for the treatment of metabolic disorders, including such disorders which are single gene disorders, and disorders which are a result of one more mutations within the lipoprotein lipase gene and ocular disorders <b>Class 5:</b> Viral systems, gene therapy systems, nucleic acid delivery systems, viral vectors, non-viral vectors, gene therapy vectors, nucleic acid delivery vectors, and cells transformed by viral vectors, non-viral vectors, gene therapy vectors or nucleic acid delivery vectors for medical purposes; pharmaceutical products; biological preparations for use in medical and clinical	8640609	5/10/2010



gene therapy, nucleic acid-based therapy and cell therapy;  
clinical medical reagents for use in nucleic acid-based therapy,  
gene therapy, cell therapy, gene diagnosis and gene testing;  
pharmaceutical preparations, vaccines, prophylaxis products  
and other products for use in nucleic acid-based therapy, gene  
therapy and cell therapy

**Class 44:** Gene and nucleic acid delivery, gene and nucleic acid transfer, gene and nucleic acid regulation and gene and nucleic acid modulation for the treatment of metabolic disorders, ocular disorders, diseases of the nervous system, blood disorders, liver disorders, muscular disorders, muscular skeletal disorders, cancers, infectious diseases, inflammatory and autoimmune diseases, vascular disorders, inherited disorders, genetic disorders and single gene disorders.

Trademark	Type	Country	goods/services	Reg. No.	Reg. Date
UNIQUIRE	Word	EU	<p><b>Class 1:</b> Viral systems, gene therapy systems, nucleic acid delivery systems, viral vectors, non-viral vectors, gene therapy vectors, nucleic acid delivery vectors, and cells transformed by viral vectors, non-viral vectors, gene therapy vectors and nucleic acid delivery vectors for non-medical research purposes</p> <p><b>Class 5:</b> Viral systems, gene therapy systems, nucleic acid delivery systems, viral vectors, non-viral vectors, gene therapy vectors, nucleic acid delivery vectors, and cells transformed by viral vectors, non-viral vectors, gene therapy vectors or nucleic acid delivery vectors for medical purposes; pharmaceutical products; biological preparations for use in medical and clinical gene therapy, nucleic acid-based therapy and cell therapy; clinical medical reagents for use in nucleic acid-based therapy, gene therapy, cell therapy, gene diagnosis and gene testing; pharmaceutical preparations, vaccines, prophylaxis products and other products for use in nucleic acid-based therapy, gene therapy and cell therapy</p> <p><b>Class 42:</b> Research, product development and consultancy in the field of biotechnology, biologics, pharmaceuticals, medical science, chemistry and biochemistry</p> <p><b>Class 44:</b> Gene and nucleic acid delivery, gene and nucleic acid transfer, gene and nucleic acid regulation and gene and nucleic acid modulation for the treatment of metabolic disorders, ocular disorders, diseases of the nervous system, blood disorders, liver disorders, muscular disorders, muscular skeletal disorders, cancers, infectious diseases, inflammatory and autoimmune diseases, vascular disorders, inherited disorders, genetic disorders and single gene disorders.</p>	10431005	4/25/2012

UniQure is also the owner of the other trademark registrations and applications in the Territory identified in Schedule 2.2(a).

#### Basic Trademark Rules:

- **The most prominent use** (which is usually the first use of our registered trademarks in a title, heading, and text of a document) should have the superscripted registered trademark symbol ®. If that symbol is not available, then use (R).
- **Distinguish our trademarks.** Visually set off our trademarks from the surrounding text.
- **Our trademarks are never plural or possessive.**
- **Never modify the form of our trademarks**, whether the trademarks are an acronym, word, words or graphic design. Unless otherwise specifically permitted in writing, word trademarks should not be modified by abbreviations, translations or connections (e.g., by a hyphen or otherwise) to other words or trademarks. Our trademarks should not be split over any lines. All logos should be reproduced in strict compliance with the established graphical form.
- **Colors of our trademarks.** The preferred treatment for the company logo is as follows:



The logo has to be displayed in printed letters. Only the “Q” is in upper case.

- **Attribute our trademarks** by properly acknowledging our ownership interest in them (e.g., “Glybera is a trademark or registered trademark of uniQure IP B.V.”). Such attribution statement may appear in any conventional location within a document or packaging (e.g., header, footer, footnote, etc.).
- **Logo, size and proportion treatments.** The “Glybera” word and device mark and the “G” device mark (logos) set forth in the table above must always be reproduced exactly as pictured in the table above, in the specific typefaces shown. No other typefaces are permitted. The logos can be reproduced in color or black& white and may be proportionately enlarged or reduced so long as legibility is ensured. uniQure reserves the right to introduce specific requirements as to the color and font size of its trademarks and logos. The logos are independent trademarks and should not be incorporated into other trademarks, logos, and artwork. The logos may appear in proximity to a licensee’s trademarks and logos and other artwork, but with a clear visual separation.
- **Inquiries regarding the Trademark Guidelines.** In case of inquiries regarding these Trademark Guidelines uniQure may be contacted at the following address: Meibergdreef 61, 1105 BA Amsterdam, The Netherlands.

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**SCHEDULE 2.3**  
**Definition**  
**Fully Loaded Cost of Goods**

Item per <b>[**]</b> batch	Costs* <b>[EUR]</b>
Clean room occupancy	<b>[**]</b>
Cell bank vial	<b>[**]</b>
Virus banks vials	<b>[**]</b>
Raw materials	<b>[**]</b>
External release assays (QC)	<b>[**]</b>
External QP	<b>[**]</b>
Personnel (MF, QC, QA)	<b>[**]</b>
Packaging (incl. release)	<b>[**]</b>
Stability study batch allocation	<b>[**]</b>
Total	<b>[**]</b>

Norms:

Number of patients per batch **[\*\*]**

Batch success rate **[\*\*]**%

Result Fully loaded costs:

<b>COG per patient</b>	<b>EUR</b>	<b>[**]*</b>
<b>COG per batch</b>	<b>EUR</b>	<b>[**]*</b>

COG relevant for Glybera Manufacturing Cost Reimbursement:

<b>COG per patient</b>	<b>EUR</b>	<b>[**]*</b>
<b>COG per batch</b>	<b>EUR</b>	<b>[**]*</b>

\* = as of the Effective Date

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**SCHEDULE 2.4(d)**  
**Minimum Order Quantity**

SCHEDULE 2.6  
Technology Transfer

The following is a non-exhaustive list describing key steps which the Parties would typically envisage for a transfer of the Manufacturing of the Product to another manufacturing site:

Steps	Estimated Timelines
· if transferred to a Third Party manufacturer: select and contract manufacturer party	[**]
· tech transfer (on paper)	[**]
· obtain time slot	[**]
· process validation at CMO	[**]
· file type II variation	[**]
· review and approval type II variation	[**]
Total	[**]

SCHEDULE 3.1  
MA/MAA Filing and Maintenance Activities and Fees Template

State	Activity	Fee Allocation	Responsible Party	Timeline
Albania				
Andorra				
Bosnia and Herzegovina				
Croatia				
Macedonia				
Monaco				
Montenegro				
Republic of San Marino				
Serbia				
Switzerland				

State	Activity	Fee Allocation	Responsible Party	Timeline
Vatican City				
Algeria				
Armenia				
Azerbaijan				
Belarus				
Brazil				
China				
Egypt				
Georgia				
Kazakhstan				
Kirghizstan				

State	Activity	Fee Allocation	Responsible Party	Timeline
Mexico				
Moldova				
Morocco				
Pakistan				
Russia				
Tajikistan				
Tunisia				
Turkey				
Turkmenistan				
Ukraine				
Uzbekistan				

State	Activity	Fee Allocation	Responsible Party	Timeline

### SCHEDULE 3.2 Regulatory Plan

A. For the EU Member States:

1. Quality

1.1 Specific Obligations

The following obligations were included in the Day 210 Assessment Report with the requirement to submit them as Type II variations prior to implementation.

Source	Name	Description	Due date	Status	Progress
D210 AR	II/003-SO1	Newly developed release assay for cellular SF+ DNA submitted and approved by a Type II variation.	[**]	[**]	[**]
D210 AR	II/004-SO2	An improved or newly developed release assay for SF+ protein or a combined SF+/Baculovirus protein approved by a Type II variation	[**]	[**]	[**]
D210 AR	II/004-SO3	Assays for Rep and Cap genes will be validated and acceptance criteria for accuracy and precision will be justified in the validation report.	[**]	[**]	[**]
D210 AR	II/001-SO4	To complete the validation of the residual infectious baculovirus assay ([**] wells), the LOD should be experimentally confirmed, in addition the presented risk assessment should be revised taking into account the experimentally determined LOD.	[**]	[**]	[**]
D210 AR	II/002-SO5	An improved release assay for replication competent AAV should be submitted and approved via a Type II variation	[**]	[**]	[**]
D210 AR	II/Oxx—SO6	To improve the virus safety profile of the product, an additional manufacturing process step should be developed	[**]	[**]	

Source	Name	Description	Due date	Status	Progress
D210 AR	II/Oxx-SO7	Additional manufacturing process step validated to ensure that the process is capable, of inactivating or removing at least the maximal baculovirus load used in production.	[**]	[**]	

The baseline eCTD Module 3 has been submitted on [\*\*].

1.2 Commitments

The commitments listed below have been made during the review and authorisation procedure. For those commitments accompanied in the D120 or D180 responses by a planned date for completion, the definite completion and submission dates have to be submitted in a Letter of Undertaking. For some commitments no completion date was mentioned. Those will not be mentioned in the Letter of Undertaking and will be considered at a later stage during the yearly product quality review.

Source	Name	Description	Due date	Status
D120Q#60 and D180Q#20, 21	LU3	Stability study on the first batch of drug substance stored in the new container is recommended to be performed prior to product being released for patient treatment.	[**]	[**]
D120Q#2 and D180Q#1	LU5	Assess how long the baculovirus sequences are persisting in vivo in mice	[**]	[**]
D120Q#2 and D180Q#1	LU6	Further characterization by N6S and analysis of [**] new batches by NGS	[**]	[**]
D120Q#71 and D180Q#24	LU1	Review and revise the specification for the ratio of full to infectious virus particles based on data from [**] batches to allow meaningful specifications to be set.	[**]	[**]
D120Q#84 and D180Q#28	LU4	To provide details on the outcome of the further development, validation and proposed implementation of the S2 cell line that is non-permissive to baculovirus.	[**]	[**]
D120Q#59	LU7	Review specifications for protein impurity 2 when data of [**] batches are available	[**]	[**]

1.3 Other variations

Variation	Description	Planned submission date	Status
II/007	New test method for residual V <sub>H</sub> H	[**]	[**]
II/008	Improved test method for residual LPL	[**]	[**]
II/009	New test method for infectious vector titre	[**]	[**]
II/Oxx	Total to Full particles ratio specification to be adapted (lower limit removed)	[**]	[**]
Tbd*	Changes to the stability protocol to remove sterility testing at the end of shelf life. A new container closure study is to be initiated	[**]	[**]

Confidential Materials omitted and filed separately with the Securities and Exchange Commission. A total of one page was omitted. [\*\*]

B. For the Territory outside of the EU Member States:

The Parties shall agree upon the Regulatory Plan for any remaining countries of the Territory outside of the EU Member States in the first meeting of the JCC after the Effective Date.

SCHEDULE 7.3  
Existing Third Party Licenses

Overview:

Licensor	Title of Agreement	Date	Relevant Intellectual Property Rights (including quality of license, i.e. exclusive / non-exclusive rights)
Xenon Pharmaceuticals Inc. (formerly Xenon Genetics Inc.)	Sublicense and research agreement between Xenon Genetics Inc. and Amsterdam Molecular Therapeutics BV	June 18, 2001	Exclusive sublicensable, not further sublicensable without written consent
US Public Health Service (PHS, NIH, HHS)	Public Health Service Patent License Agreement - Nonexclusive	May 2, 2007	Non-exclusive, sub-licensable upon written approval prior review
Ampliphi Biosciences Corporation (legal	License agreement	December 5, 2006 March 12, 2012	Non-exclusive, non-sublicensable

successor of Targeted Genetics)	Amendment No1 to the license agreement		
Aventis Pharma S.A.	License agreement	December 20, 2006	Exclusive, non-transferable and non-assignable

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Salk Institute for Biological Studies	License agreement between Salk Institute for Biological Studies and Amsterdam Molecular Therapeutics BV, RNA export element WPRE	February 8, 2008	Non-exclusive, non-transferable
Asklepios Biopharmaceutical Inc. (AskBio)	Non-exclusive license agreement	September 3, 2010	Non-exclusive, sublicensable
Protein Sciences Corporation	License agreement — non-exclusive ( <i>express</i> SF+ cells)	March 22, 2007	Non-exclusive, non-sublicensable
	License agreement — non-exclusive ( <i>express</i> SF+ cells) - AMENDMENT	June 13, 2012	Exclusive (with respect to Product), sublicensable

#### Text of Agreements:

[full text of agreements to be provided to Chiesi on CD-ROM within [\*\*] Business Days after signing of this Agreement]

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### **SCHEDULE 8.1(a)** **Commercially Reasonable Efforts**

At all times during the Term Chiesi shall in no event use less than Commercially Reasonable Efforts to Commercialize the Product as further described in this Schedule 8.1(a). Such efforts shall include:

#### Compliance with Marketing Plan and Budget

Chiesi shall perform all activities related to the Commercialization of the Product set forth in the marketing plan and budget to be agreed upon between the Parties annually (the “Marketing Plan”). The Marketing Plan shall require Chiesi to perform promotional activities typically applied in the pharmaceutical industry for orphan drugs, to draw the attention and interest to the Product and to render technical support to explain the efficacy of the Product, including detailing and training physicians, pharmacists or other prescribers. The detailing of the Products shall include: (a) regular visits and calls made to physicians and pharmacists by Chiesi’s marketing/detailing staff to provide Product information; and (b) activities implemented to call the attention of physicians, pharmacists and other prescribers such as organizing conferences, seminars, physicians- and pharmacists training sessions(including responsibility of the risk management plan (RMP) required with training materials in local language), lectures, mailings with announcements and product brochures, publications in professional magazines, and participation in trade exhibitions or symposia, subject to the requirements and limitations of any Applicable Laws.

The Marketing Plan shall further include details on the responsibility of Chiesi for (a) the provision and review of marketing materials, (b) training and maintaining a sufficient number of suitably qualified sales force, (c) training and maintaining a sufficient number of suitably qualified medical personnel, (d) collection and conveyance to uniQure of general market data (including customer requirements with respect to the Product; market analysis; and competition.

The Marketing Plan for the first year of the Agreement, attached hereto as Appendix A, sets forth the obligations of Chiesi in connection with the First Commercial Sale of the Product in the Territory.

#### Allocation of Minimum FTEs

Chiesi shall allocate a minimum number of FTEs in the Territory as follows (“Minimum FTEs”):

- 1<sup>st</sup> anniversary after the Effective Date: [\*\*] FTEs
- 2<sup>nd</sup> anniversary after the Effective Date: [\*\*] FTEs
- 3<sup>rd</sup> anniversary after the Effective Date: [\*\*] FTEs
- 4<sup>th</sup> anniversary after the Effective Date: [\*\*] FTEs
- 5<sup>th</sup> anniversary after the Effective Date: [\*\*] FTEs

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Beginning in 2013, prior to [\*\*] of each year, Chiesi shall submit the Marketing Plan for any subsequent years to uniQure for its review and approval, which shall not be unreasonably withheld or delayed.

#### Setting of Target Price

##### First Submission (Germany) by uniQure

Notwithstanding the provisions hereunder, but without prejudice to Section 4.1(f)(ii), the Parties hereby agree that uniQure shall submit a dossier to the competent reimbursement body in Germany (GBA) before end 2013, which shall include a target manufacturer selling price (*Herstellereingangspreis*) (the “Target Price”) of no less than EUR [\*\*]. In case upon further investigation Chiesi is of the opinion that such Target Price in Germany can only be set at a lower level, Chiesi shall start consultations with uniQure on such lower Target Price at least [\*\*] prior to uniQure submitting the dossier to the competent reimbursement body with the goal of both Parties agreeing to a lower Target Price mutually acceptable to the Parties. If the Parties cannot agree on such mutual lower Target Price before the end of such [\*\*] period, uniQure shall submit the dossier to the competent reimbursement body with a Target Price of at least EUR [\*\*]. The Target Price submitted to the German GBA is to be used as reference price in the other European countries mentioned below and hence such Target Price shall be used for all submissions by Chiesi in such other European countries.

In line with the Marketing Plan, Chiesi shall start with the Commercialization of the Product in Germany irrespective of the competent reimbursement body having rendered its final decision, provided all other requirements to start Commercializing the Product in Germany have been fulfilled.

#### Timelines for Price and Reimbursement Submissions in other European countries by Chiesi, unless otherwise agreed upon between the Parties in due course

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Confidential Materials omitted and filed separately with the Securities and Exchange Commission. A total of 42 pages were omitted. [\*\*]

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#### **SCHEDULE 8.1(b) Approved Activities**

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#### **SCHEDULE 9.1 Upstream Licenses Relevant for Condition Precedent**

<b>Licensor</b>	<b>Title of Agreement</b>	<b>Date</b>	<b>Note</b>
Xenon Pharmaceuticals Inc. (formerly Xenon Genetics Inc.)	Sublicense and research agreement between Xenon Genetics Inc. and Amsterdam Molecular Therapeutics BV	June 18, 2001	Exclusive sublicensable, not further sublicensable without written consent
US Public Health Service (PHS, NIH, HHS)	Public Health Service Patent License Agreement - Nonexclusive	May 2, 2007	Non-exclusive, sub-licensable upon written approval prior review
Ampliphi Biosciences Corporation (legal successor of Targeted Genetics)	License agreement	December 5, 2006	Non-exclusive, non-sublicensable
Aventis Pharma S.A.	Amendment No1 to the license agreement	March 12, 2012	
	License agreement	December 20, 2006	Exclusive, non-transferable and non-assignable
Salk Institute for Biological Studies	License agreement between Salk Institute for Biological Studies and Amsterdam Molecular Therapeutics BV, RNA export element WPRE	February 8, 2008	Non-exclusive, non-transferable

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Note:

The Parties agree that the condition precedent may be fulfilled not only by subcontracting of the rights and licenses licensed by uniQure as licensee under the Existing Third Party Licenses but also by equivalent arrangements mutually agreed between the Parties and the respective Third Party licensor (for instance in cases, where the Third Party licensor (such as the Salk Institute for Biological Studies) is of the opinion that a sublicense is not required for the activities performed by Chiesi, its Affiliates and Sub-distributors in connection with this Agreement).

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Confidential Materials omitted and filed separately with the Securities and Exchange Commission. Double asterisks denote omissions.

# LICENSE AGREEMENT

by and among

FUNDACIÓN PARA LA INVESTIGACIÓN MÉDICA APPLICADA (1)

PONTEGADEA BIOTECHNOLOGICA, S.L. SOCIEDAD DE DESAROLLO DE NAVARA, S.A., CIERVANA, S.L., CINAMAR, S.A, MASAVEU DE INVESTIGACIONES Y DESAROLLO, S.L., INVESTIGACIONES 2001 CORPCAN, S.L., ALAZADY BIOTECNOLÓGICA, S.L., SOCIEDAD ANDALUZA DE INVESTIGACIÓN DE LA SALUD, S.L.U., INSTITUTO DE EDUCACIÓN E INVESTIGACIÓN S.A, LOYALTY SQUARE, S.L., UNICARTERA CAJA 2, S.L., CAJA RURAL DE NAVARRA, SOCIEDAD COOPERATIVA LIMITADA DE CREDITO, UNGRIA PATENTES Y MARCAS, S.A., FUERTES I MÁS D, S.L., GAINMÉDICA, S.L., UNIÓN TEMPORAL DE EMPRESAS LEY 18/1982, DE 26 MAYO NÚM 1.334/2003 (2)

PROYECTO DE BIOMEDICINA CIMA S.L. (3)

DIGNA BIOTECH, S.L. (4)

AND

AMSTERDAM MOLECULAR THERAPEUTICS (AMT) B.V. (5),

dated as of 21<sup>st</sup> May, 2010

# LICENSE AGREEMENT

THIS LICENSE AGREEMENT dated as of 21 May, 2010 (the “Agreement”) is made by and among:

- (1) Fundación para la Investigación Médica Aplicada (“FIMA”) incorporated under the laws of Spain, by means of the public deed executed on December 10th, 1998, before the Notary Public of Madrid, Víctor Manuel Garrido de Palma, under the number 3001 of its protocol; with Tax Identification Number G82198524 and with registered offices at Calle Pintor Paret, 5, 1 F, Pamplona, Spain; duly represented by Mr Francisco Errasti Goenaga;
- (2) Pontegadea Biotecnologica, S.L, Sociedad de Desarrollo de Navarra, S.A., Ciervana, S.L., Cinamar, S.A., Masaveu de Investigaciones y Desarrollo, S.L., Investigaciones 2001 CORPCAN, S.L., Alazady Biotecnológica, S.L., Sociedad Andaluza de Investigación de la Salud, S.L.U., Instituto de Educación e Investigación S.A., Loyalty Square, S.L., Unicarera Caja 2, S.L., Caja Rural de Navarra, Sociedad Cooperativa Limitada de Credito, Ungria Patentes y Marcas, S.A., Fuertes I Más D, S.L., Gainmédica, S.L., Unión Temporal de Empresas Ley 18/1982, de 26 de Mayo, Núm. 1.334/2003, (the collaborative research consortium known as “**UTE CIMA**”), incorporated under the laws of Spain, by means of the public deed executed on June 3<sup>rd</sup>, 2003, before the Notary Public of Pamplona, Jose Javier Castiella Rodriques, under the number 1461 of its protocol, with Tax Identification Number G31790595 and with registered offices at Avda. De Carlos III, 36, 1 Dcha, Pamplona, Spain; duly represented by Mr Antonio Martin Catón;
- (3) Proyecto de Biomedicina CIMA S.L. (“**Proyecto**”) with corporate address at Avda. Carlos III. 36, 1 dcha., 31003 Pamplona, Navarra, Spain; duly represented by Mr Antonio Martin Canton;
- (4) Digna Biotech, S.L. (“**Digna**”) with corporate address at C/ Etxesakan 28, oficina 5, 31180 Cizur Maryo, Navarra, Spain, bearer of Tax Identification Number B-31778509, duly represented by Mr Pablo Ortiz Bétes;
- (5) Amsterdam Molecular Therapeutics (AMT) B.V. (“**AMT**”) a company with limited liability incorporated under the laws of The Netherlands with registered office at Meibergdreef 61, NL-1105 BA Amsterdam, The Netherlands, duly represented by Mr. Piers Morgan;

## WHEREAS

(A) FIMA and UTE CIMA collaborate in a project called “CIMA Project” for medical investigation and research. On June 3<sup>rd</sup>, 2003, FIMA and UTE CIMA entered into a Investigation Contract in the frame of a contractual joint venture (“**Joint Venture**”) by virtue of which FIMA, in consideration for the remuneration agreed between the parties, carries out those investigations required for the fulfillment of the purpose of CIMA Project, with the results derived from said investigations by FIMA being owned solely and automatically by UTE CIMA;

(B) Pursuant to the Joint Venture, UTE CIMA undertook to assign to Proyecto, all of UTE CIMA’s right, title and interest in any results derived from the above investigations, when such results might be subject to protection and/or exploitation according to the intellectual property regulations;

(C) Proyecto has an agreement with Digna dated June 14<sup>th</sup>, 2005 under which Digna undertakes to carry out on behalf of Proyecto prosecution, maintenance, enforcement and defense tasks of the above intellectual property. In addition, Proyecto has entered into an agreement with Digna dated June 25<sup>th</sup>, 2005, amended on December 20<sup>th</sup>, 2005, under which Digna is entitled to an exclusive worldwide license to develop and commercialize the above results;



(D) AMT is a biopharmaceutical company that owns or controls Patents, Know-How and Materials relating to the research, development, registration, manufacture and commercialization of therapies based on constructs including adeno-associated virus ("AAV") vectors;

(E) In July 2005, FIMA, UTE CIMA and AMT entered into an agreement ("**2005 Agreement**") for the conduct of a collaborative research and development program to construct an AAV vector which could be used in the therapy of porphyria which was since extended to include research and development into reducing the immunogenicity of the AAV therapies. In anticipation of the successful generation of an AAV vector which could be used in the therapy of porphyria, the parties discussed potential opportunities for the further development and commercialization of Products (as defined in the 2005 Agreement);

(F) On May 1, 2007, the Parties entered into a Commercialization Agreement ("**2007 Commercialization Agreement**") pursuant to which, inter alia, (i) Digna relinquished its rights to develop and commercialize Products (as defined in the 2007 Commercialization Agreement) under such Intellectual Property to facilitate the 2007 Commercialization Agreement, (ii) AMT was granted exclusive rights to develop and commercialize Products (as defined in the 2007 Commercialization Agreement) and (iii) the 2005 Agreement was terminated;

(G) On July 25<sup>th</sup>, 2007, AMT, FIMA, UTE CIMA, PB CIMA and Digna entered into an agreement to give AMT and its Affiliates privileged access to the results of the research being conducted by or upon behalf of UTE CIMA and FIMA ("**Privileged Access Agreement**") with an option for AMT to participate in the Development of Candidate Products (as those terms are defined in the Privileged Access Agreement) and, if this yielded positive results for AMT or its Affiliates to have the right to take an exclusive license under relevant intellectual property to further research, develop, make, register and commercialize such Candidate Product;

(H) In relation to the Candidate Product Virus encoded IGF-1 for the treatment of liver cirrhosis, AMT exercised its option to take an exclusive license pursuant to which Digna, Proyecto and AMT entered into a license agreement dated November 9<sup>th</sup>, 2007 (the "**Virus encoded IGF License**");

(I) The Parties now wish to modify their collaboration under aforementioned agreements and to refocus their efforts and resources to the further development and commercialization of a gene therapy treatment for acute intermittent porphyria under the terms and conditions set forth below;

## IT IS NOW AGREED AS FOLLOWS

### ARTICLE 1 DEFINITIONS

For purposes of this Agreement, the terms defined in this Article 1 shall have the meanings specified below. Certain other capitalized terms are defined elsewhere in this Agreement.

1.1 "**Affiliate**" any company, partnership or other business entity which Controls, is Controlled by or it under common Control with any of the Parties. For the purposes of this definition "Control" refers to any of the following (i) the possession, directly or indirectly, of the power to direct the management or policies of an entity, whether through ownership of voting securities, by contract or otherwise; (ii) ownership of fifty percent (50%) or more of the voting securities entitled to vote for the election of directors in the case of a corporation, or of fifty percent (50%) or more of the equity interest in the case of any other type of legal entity; (iii) status as a general partner in any partnership, or any other arrangement whereby a Party controls or has the right to control the board of directors or equivalent governing body of a corporation or other entity.

1.2 "**Agreement**" or "License Agreement" means this License Agreement.

1.3 "**AMT Background IP**" means any IP owned or Controlled by AMT prior to the Effective Date or developed or acquired by AMT after the Effective Date, but excluding Joint Patent Rights and excluding any IP that has been developed prior to the Effective Date under the collaborative research programs jointly carried out by the Parties. AMT Background IP does, however include manufacturing knowhow developed by AMT within or outside said collaborative research programs.

1.4 "**Calendar Quarter**" means each period of three months ending on 31 March, 30 June, 30 September or 31 December and "Quarterly" shall be construed accordingly.

1.5 "**Calendar Year**" means each successive period of twelve calendar (12) months commencing on 1 January.

1.6 "**CIMA Parties**" means FIMA, UTE CIMA, Proyecto and Digna jointly and a "CIMA Party" means FIMA, UTE CIMA, Proyecto or Digna.

1.7 "**CIMA Background IP**" means any IP owned or Controlled by a CIMA Party at the Effective Date or developed or acquired by a CIMA Party after the Effective Date outside the scope of this Agreement and/or the Collaborative Development Agreement and that is useful for the development or Commercialization of the Product, but excluding Joint Patent Rights and excluding any IP that has been developed prior to the Effective Date under the collaborative research programs jointly carried out by the Parties.

1.8 "**Collaborative Development Agreement**" means the agreement between Digna and ATM attached hereto as **Exhibit 2**.

1.9 "**Commercialization**", "**Commercializing**" or "**Commercialize**" means all activities relating to the import, advertising, promotion and other marketing, pricing and reimbursement, detailing, distribution, storage, handling, offering for sale and selling, customer service and support, post Regulatory Approval regulatory activities including phase IV clinical trials and adverse event reporting in relation to the Product.

1.10 "**Commercially Reasonable Efforts**" means in respect of AMT efforts and resources commonly used by biotechnology companies of a similar size to AMT based on funds raised.

1.11 "**Confidential Information**" means, subject to the exceptions set forth in Article 8.3 (i) the terms and conditions of this Agreement and the Collaborative Development Agreement, for which each Party will be considered a Disclosing Party and a Recipient Party; and (ii) any non-public information, whether or not patentable, disclosed or provided by one Party to the other Party in connection with this Agreement, including, without limitation, any information

which release is likely to prejudice the commercial interests of the parties, or is considered as a trade secret, including information regarding such Party's strategy, business plans, objectives, research, technology, products, business affairs or finances including any non-public data relating to development or Commercialization of any Product and other information of the type that is customarily considered to be confidential information by parties engaged in activities that are substantially similar to the activities being engaged in by the Parties under this Agreement, for which the Party making such disclosure will be considered the Disclosing Party and the receiver will be the Recipient Party.

1.12 "Control" (including variations such as "Controlled") means with respect to any Intellectual Property, possession of the right, whether directly or indirectly, and whether by ownership, license or otherwise, to assign, or grant a license, sub-license or other right to or under, such Intellectual Property without violating the terms of any agreement or other arrangement with any Third Party.

1.13 "Cover", "Covered" or "Covering" means, with respect to a Patent Right that, but for a license under an issued Valid Claim included in such Patent Right, the manufacture, use, transportation, sale, offer for sale, or importation of the Product would infringe such Valid Claim or, in the case of a Patent Right that is a patent application, would infringe a Valid Claim in such patent application if it were to issue as a patent.

1.14 "Disclosing Party." means the Party which discloses Confidential Information to the other Party or Parties.

1.15 "Disorder" means acute intermittent porphyria.

1.16 "Documents" means analyses, books, CD-ROM, USB stick, charts, comments, computations, designs, discs, diskettes, files, graphs, ledgers, notebooks, paper, photographs, plans, records, recordings, reports, research notes, tapes and other graphic or written data or other media and other computer information storage means on which Know How is permanently stored and advertising and promotional materials of any nature whatsoever including preparatory materials for the same.

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1.17 "Effective Date" means the date first set forth above.

1.18 "Gene Therapy Field" means the use of genetic material in any of the following ways for the treatment or prophylaxis of a disease (a) delivery of a functional version of a mutant gene or any other DNA that encodes for a therapeutic molecule into the nucleus or mitochondria of patient's cells (b) by the insertion of a normal gene into a non-specific location within the genome to replace a non-functional gene; or (c) by swapping an abnormal gene for a normal gene (through homologous recombination); or (d) by repairing an abnormal gene through selective reverse mutation which returns the gene to its normal function; or (e) by altering the regulation (the degree to which a gene is turned on or off) of a particular gene.

1.19 "Intellectual Property" or "IP" means Patent Rights, Know How and/or Materials.

1.20 "Joint Patent Rights" means the patent application set forth in Exhibit 1 that is filed in the names of Amsterdam Molecular Therapeutics (AMT) IP B.V. and Proyecto de Biomedicina CIMA S.L jointly and any and all Patent Rights deriving from said patent application anywhere in the world.

1.21 "Know-How" means technical and other information which is not in the public domain, including information comprising or relating to concepts, discoveries, data, designs, formulae, ideas, inventions, Materials, methods, models, research plans, procedures, designs for experiments and tests and results of experimentation and testing (including results of research or development) processes (including manufacturing processes, specifications and techniques), laboratory records, chemical, pharmacological, toxicological, clinical, analytical and quality control data, clinical and non-clinical trial data, case report forms, data analyses, reports, manufacturing data or summaries and information contained in submissions to and information from ethical committees and Regulatory Authorities. Know How includes Documents containing Know How, including but not limited to any rights including trade secrets, copyright, database or design rights protecting such Know How. The fact that an item is known to the public shall not be taken to preclude the possibility that a compilation including the item, and/or a development relating to the item, is not known to the public.

1.22 "Launch" means the first arms-length commercial sale to a Third Party of the Product by AMT, its Affiliates or sub-licensees after grant of required Regulatory Approval and after pricing or reimbursement approval has been granted (if required in that country). Sales for test marketing, clinical trial purposes or compassionate or similar use do not constitute a Launch.

1.23 "Losses" means any and all losses, damages, liabilities, costs and expenses (including, without limitation, reasonable attorneys' fees and expenses). In calculating "Losses", the duty to reasonably mitigate on the part of the Party suffering the Losses shall be taken into account.

1.24 "Materials" means any chemical or biological substances including but not limited to blood samples, nucleotide or nucleotide sequence including DNA and RNA sequences, genes, vector or construct including plasmids, phages or viruses, host organism including bacteria, fungi, algae, protozoa and hybridoma's, eukaryotic or prokaryotic cell line or expression system or any development strain or product of that cell line or expression system,

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protein including any peptide or amino acid sequence, enzyme, antibody or protein conferring targeting properties and any fragment of a protein or a peptide enzyme or antibody, assay or reagent, any other genetic or biological material or micro-organism.

1.25 "Net Revenues" means:

(i) Any signature fee or other up-front fee due to be received by AMT or any of its Affiliates from a licensee being appointed by AMT or any of its Affiliates to further develop and/or Commercialize a Product; and

(ii) Any milestone or other payments due to be received by AMT or any of its Affiliates from a licensee being appointed by AMT or any of its Affiliates to further develop and/or Commercialize a Product which payments are payable on an event to occur in relation to the development or Commercialization thereof but always excluding sums received by AMT or any of its Affiliates from such licensee which reimburse AMT for the cost and expense (but only the cost and expenses and no profit element) of research or development work to be undertaken by or upon behalf of AMT of its Affiliate directly related to the development of Product; and

(iii) Any sums due to be received by AMT or any of its Affiliates from a licensee appointed by AMT or any of its Affiliates to Commercialize a Product which sums are calculated by reference to the sales volumes of the Product by such licensee as a percentage of a net sales or similar definition, but always excluding any sums received by AMT or its Affiliates for sales of Products by AMT or Affiliates to distributors or other Third Parties, provided that those sums are accounted as “Net Sales”.

In the event that AMT, or its Affiliate, receives non-monetary consideration from a licensee to further develop and/or Commercialize a Product, Net Revenues shall be calculated based on the fair market value of such consideration.

1.26 “Net Sales” means the gross amount invoiced for sales of Product, in arm’s length sales by AMT or its Affiliates to Third Parties, less the following deductions from such gross amounts which are actually incurred, allowed, accrued or specifically allocated:

- (i) normal and customary trade cash and quantity discounts actually given, credits, price adjustments or allowances for damaged Products, returns or rejections of Products;
- (ii) chargeback payments and rebates (or the equivalent thereof) for Product granted on a customary trade basis to group purchasing organizations, managed health care organizations or to federal, state/provincial, local and other governments, including their agencies, or to trade customers;
- (iii) reasonable and customary freight, shipping insurance and other transportation expenses directly related to the sale of Product (if actually borne by AMT or its Affiliates without reimbursement from any Third Party);
- (iv) required distribution commissions/fees payable to any Third Party providing distribution services to AMT;

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(v) sales, value-added, excise taxes, tariffs and duties, and other taxes and governmental charges directly related to the sale, to the extent that such items are included in the gross invoice price of Product and are actually borne by AMT, its Affiliates, without reimbursement from any Third Party (but not including taxes assessed against the income derived from such sale); and

(vi) actual uncollectible amounts for Product where collectability is determined in accordance with IFRS consistently applied to all AMT products.

In the event that AMT, or its Affiliate, receives non-monetary consideration from a Third Party for sale of Product, Net Sales shall be calculated based on the fair market value of such consideration.

1.27 “Parties” means the parties to this Agreement and “Party” means a party to this Agreement.

1.28 “Patent Rights” means a (i) all national, regional and international patents and patent applications, including provisional patent applications, (ii) all patent applications filed either from such patents, patent applications or provisional applications or from an application claiming priority from any of these, including divisional, continuations, continuations-in-part, provisions, convened provisionals and continued prosecution applications, (iii) any and all patents that have issued or in the future issue from the foregoing patent applications (i) and (ii), including author certificates, inventor certificates, utility models, petty patents and design patents and certificates of invention, (iv) any and all extensions or restorations by existing or future extension or restoration mechanisms, including revalidations, reissues, re-examinations and extensions (including any supplementary protection certificates and the like) of the foregoing patents or patent applications (i), (ii) and (iii), and (v) any similar rights, including so-called pipeline protection, or any importation, revalidation, confirmation or introduction patent or registration patent or patent of additions to any such foregoing patent applications and patents.

1.29 “Product” means any product that is, or that utilizes technology Covered by the Joint Patent Rights.

1.30 “Previous Agreements” means the 2005 Agreement, the Privileged Access Agreement, the Virus encoded IGF License and the 2007 Commercialization Agreement jointly.

1.31 “Receiving Party” means any Party receiving Confidential Information from another Party;

1.32 “Regulatory Approval” means all approvals from Regulatory Authorities in any country in the Territory required lawfully to develop, clinically test, manufacture and market the Product in any such country, any establishment license application filed with the FDA or other Regulatory Authority to obtain approval of the facilities and equipment to be used to manufacture a Product, any Investigational New Drug or other investigational filing, including but not limited to any authorization for the import, manufacture and clinical testing of the Product (whether or not in filled and finished form), and any product pricing approvals where applicable.

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1.33 “Regulatory Authority” means any relevant national (e.g., the FDA, EU member states authorities), supra-national (e.g., the European Commission, the Council of the European Union, or the EMEA), or other relevant governmental entity in any jurisdiction of the world involved in the granting of Regulatory Approvals for pharmaceutical product.

1.34 “Territory” means the world.

1.35 “Third Party” means a party other than any of the Parties or any of their respective Affiliates.

1.36 “Valid Claim” means either a claim of (a) an issued, unexpired patent which has not been revoked or held unenforceable or invalid by a decision of a court or governmental agency of competent jurisdiction from which no appeal can be taken, or with respect to which an appeal is not taken within the time allowed for appeal, and which has not been disclaimed, denied or admitted to be invalid or unenforceable through reissue, disclaimer or otherwise, or (b) any patent application which has not been cancelled, withdrawn, or abandoned, or been pending for more than [\*\*] years from the earliest priority date claimed for such application, unless and until such claim becomes an issued claim of an issued patent.

## ARTICLE 2      **TERMINATION OF PREVIOUS AGREEMENTS**

2.1      General. The Parties agree that with effect from the Effective Date all the terms of the Previous Agreements (including but not limited to the financial obligations of AMT thereunder, whether or not already due and outstanding prior to the termination of the Previous Agreements) are terminated and to be replaced in their entirety by the terms of this Agreement, save for the clauses that will survive as set forth in Section 2.2 hereof and that each Party has no claim against each other Party in connection with the termination of the Previous Agreements agreed herein but for the surviving clauses set forth below.

2.2      Surviving clauses. The following clauses in the Previous Agreements will survive:

2.2.1      Privileged Access Agreement

- (a)      Article 5 (Confidentiality, Publicity and Press Releases)
- (b)      Article 9 (Governing Law)
- (c)      Article 10 (Jurisdiction)

2.2.2      2007 Commercialization Agreement

- (a)      Article 2 (termination of, inter alia, 2005 Agreement)
- (b)      Section 3.1 (ownership of Collaborative Research IP, as defined in the 2007 Commercialization Agreement)

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(c)      Section 3.4 (waiver and release of Digna regarding the development or Commercialization of Products (as defined in the 2007 Commercialization Agreement))

- (d)      Article 6 (Confidentiality)
- (e)      Sections 7 (Representations and Warranties)
- (f)      Article 11 (Dispute Resolution)

2.2.3      Virus Encoded IGF License

- (a)      Article 6 (Confidentiality)
- (b)      Article 10 (Governing Law)
- (c)      Article 11 (Jurisdiction)

2.3      For the avoidance of doubt: (i) as per the Effective Date of this Agreement, the license granted to AMT under Optioned IP (as defined in the Virus Encoded IGF License) shall terminate, the costs of further maintenance, prosecution, enforcement and defense of the Optioned IP shall be borne by Digna and AMT shall cease the development, manufacture and Commercialization of Virus Encoded IGF, (ii) the license granted to AMT under the 2007 Commercialization Agreement to develop and Commercialize Products (as defined in the 2007 Commercialization Agreement) is replaced by the license granted to AMT under Article 3 of this Agreement subject to the terms and conditions of this Agreement and (iii) any amount due and outstanding by AMT under the Virus Encoded IFG License is waived by the CIMA Parties with effect as from the Effective Date.

## ARTICLE 3      **OWNERSHIP AND LICENSE**

3.1      Ownership of Background. AMT is and remains the sole owner of the AMT Background IP. The CIMA Parties are and remain the sole owner of the CIMA Background IP. The Joint Patent Rights are and remain jointly owned by Amsterdam Molecular Therapeutics (AMT) IP B.V. and Proyecto de Biomedicina CIMA S.L.

3.2      Grant of Rights from the CIMA Parties to AMT. As appropriate, UTE CIMA and Proyecto hereby grant to AMT and its Affiliates:

3.2.1      an exclusive right and license, with the right to grant sublicenses, under Proyecto's interest, right and title in the Joint Patent Rights to use, develop, make, have made and Commercialize Products within the Territory; and

3.2.2      a non-exclusive, fully paid up, royalty free, right and license, with the right to grant sublicenses, under the CIMA Background IP required for the use, development, manufacture and/or Commercialization of the Product within the Territory and in the Gene Therapy Field and only to the extent required for said purpose. The CIMA Parties shall notify

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AMT in writing regularly (at least [\*\*]) on any CIMA Background IP developed after the Effective Date, describing such new developed CIMA Background IP.

3.3      Registration of license. The CIMA Parties shall upon first request of AMT cooperate in the registration of the licenses granted to AMT hereunder in the patent registers of the applicable patent offices, at AMT's expense.

3.4      Commercially Reasonable Efforts. AMT agrees to use Commercially Reasonable Efforts to further develop, manufacture and Commercialize Products as soon as reasonably practicable. If AMT does not fulfill its financial obligations under the Collaborative Development Agreement and/or in any other way does not use Commercially Reasonable Efforts to further develop, manufacture and Commercialize Products as soon as reasonably practicable,, DIGNA might revoke the license.

## ARTICLE 4 COLLABORATIVE DEVELOPMENT AGREEMENT

4.1 Development Plan. The Parties acknowledge that Digna and AMT have entered into that certain Collaborative Development Agreement of even date herewith (attached hereto as Exhibit 2, the "Collaborative Development Agreement") aimed at the further developed of a Product for treatment or prevention of acute intermittent porphyria.

## ARTICLE 5 ROYALTIES ON NET SALES AND NET REVENUES

5.1 Royalties on Net Sales. If the Product is Commercialized by AMT, AMT shall pay a [%] percent royalty on Net Sales on all Products on a Product-by-Product and country-by-country basis for the longer of the two following periods: (i) for so long as there are Valid Claims of Joint Patent Rights in such country of sale; or (ii) for the period of orphan drug marketing exclusivity granted by the applicable regulatory authority on a country by country basis, being ten (10) years in the European Union or seven (7) years in the United States post Launch of the Product, as applicable.

5.2 Royalties on Net Revenues. If the further development and/or Commercialization of a Product is licensed to a Third Party, AMT shall pay a [%] percent of Net Revenues received by AMT from any licensee under any license granted by AMT under the Joint Patent Rights for so long as such Net Revenues are received.

5.3 Payment to Digna. All royalties shall be paid to Digna. Digna shall be responsible for the apportionment of the sums paid to Digna hereunder to the respective CIMA Parties, pursuant to the agreements executed between them, and for payment of the same according to that apportionment. All other CIMA Parties agree with payment of the royalties by AMT to Digna.

5.4 Additional licenses. The Parties acknowledge that any vector license required by AMT from NIH to use, develop, make, have made or Commercialize Products will be borne by AMT. In the event that a license, sublicense or similar right from one or more Third Parties is necessary in order to make, have made, use, offer to sell, sell or import Product other than said licenses from NIH, then, upon notification to the CIMA Parties, AMT, its Affiliates or licensees may acquire such a license, sublicense or similar right and AMT may offset a total of [%]percent

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([%]) of any royalty or other payments paid in connection therewith against any royalty payments due to Digna under this Article 5; *provided, however*, that in no event shall the total royalty payable to Digna on any Product be less than [%] percent ([%] %) of the Net Sales of such Product sold by AMT, its Affiliates or licensees as a result of such off-set.

5.5 Quarterly Basis. All sums due to Digna under Sections 5.1 and 5.2 shall be calculated and payable on a Quarterly basis, and shall be paid in EURO within [%] days following the end of each Calendar Quarter. Each such payment shall be accompanied by a written report indicating the amount of Net Sales in the Territory and Net Revenues during such Calendar Quarter (including quantity of Product sold by party -i.e AMT, its Affiliates and licensees), the gross amounts that correspond to such Net Sales and Net Revenues, the currency conversion rates used (if any) and a calculation of the sums due.

5.6 Currency. Whenever for the purpose of calculating royalties conversion from any foreign currency shall be required, such conversion shall be made as follows. When calculating the Net Sales, the amount of such sales in foreign currencies shall be converted into EURO using the average monthly rate of exchange for such currencies at the time published in Financial Times in accordance with current standard practices in the market. AMT shall make all payments under this Agreement in EURO. If AMT becomes obliged by law to make a deduction or withholding in respect of tax from any amount payable under this Agreement it shall make this deduction.

5.7 Records. AMT and its Affiliates shall keep and AMT shall require its licensees to keep, full, true and accurate records and books of account containing all particulars that may be necessary for the purpose of calculating all royalties payable to Digna for a minimum period of [%] years after each payment. Upon timely notice by Digna, AMT shall permit an independent certified public accountant selected by Digna and acceptable to AMT, which acceptance shall not be unreasonably withheld, to have access during normal business hours to such records of AMT and its Affiliates as may be reasonably necessary to verify the accuracy of the royalty reports described herein. Any such certified public accountant shall first be required to enter into a confidentiality agreement in form reasonably acceptable to AMT. AMT shall use commercially reasonable efforts to schedule all such verifications within [%] days after Digna makes its written request. Such verifications shall be conducted not more than [%]. If Digna's independent certified public accountant concludes that additional royalties were owed to Digna during such period, the additional fees shall be paid by AMT within [%] days after the date Digna delivers to AMT such independent certified public accountant's written report so concluding, unless AMT shall have a good faith dispute as to the conclusions set forth in such written report, in which case AMT shall provide written notice to Digna within such [%] day period of the nature of its disagreement with such written report. In the event Digna's independent certified public accountant concludes that there was an overpayment of royalties to Digna during such period, the overpayment shall be repaid by Digna within [%] days after the date AMT received such independent certified public accountant's written report so concluding or, at the election of AMT, be credited against future royalties, unless Digna shall have a good faith dispute as to the conclusions set forth in such written report, in which case Digna shall provide written notice to AMT within such [%] day period of the nature of its disagreement with such written report. In the event a Party provides written notice of such a dispute hereunder, the Parties shall thereafter, for a period of [%] days, attempt in good faith to resolve such dispute and if they are unable to

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do so then either Party may take appropriate legal actions to enforce its rights hereunder. The fees charged by such independent certified public accountant shall be paid by Digna unless the audit discloses an underpayment of the fees payable by AMT for the audited period of more than [%] percent ([%]%) or more than [%] EURO (€ [%]), in which case AMT shall pay the reasonable fees and expenses charged by such accountant.

5.8 Taxes. All payments to Digna under the terms of the Agreement are expressed to be exclusive of value added tax howsoever arising and AMT shall pay to Digna in addition to those payments all value added tax for which Digna is liable to account in relation to any supply made or deemed to be made for value added tax purposed to this Agreement on receipt of a tax invoice or invoices from Digna.

5.9 Transfer of amounts. Payments made to Digna under this Agreement shall be made by wire transfer to the following account of Digna:

Bank: BBVA,  
Bank address: Avda. Carlos III, 33, 31004 Pamplona

or any other bank account that may be notified by Digna to AMT from time to time.

## **ARTICLE 6 FILING, MAINTENANCE AND PROSECUTION OF PATENT RIGHTS**

6.1 AMT Background IP. AMT shall have the exclusive responsibility, at its sole expense, to file, maintain, prosecute, defend and enforce Patent Rights including within the AMT Background IP, using patent counsel at its election.

6.2 CIMA Background IP. Digna or a CIMA Party designated by Digna shall have the exclusive responsibility, at its sole expense, to file, maintain, prosecute, defend and enforce Patent Rights including within the CIMA Background IP, using patent counsel at its election.

6.3 Joint Patent Rights.

6.3.1 Filing, Maintenance and Prosecution. AMT shall have the exclusive responsibility to file, maintain and prosecute the Joint Patent Rights, using patent counsel at its election. The costs thereof shall be jointly borne by Proyecto and AMT. AMT will consult with Digna, keep Digna reasonably informed and obtain the prior approval from Digna regarding the status and strategies associated with any Patent Rights included in the Joint Patent Rights. Digna, in turn, will keep the other CIMA Parties reasonably informed. If AMT elects, in its sole discretion, not to initiate or continue to pursue the further prosecution of one or more Patent Rights included in the Joint Patent Rights in any particular country, then it shall, subject to any contractual obligations to Third Parties, notify Digna in writing of such election at least [\*\*] days prior to the last available date to allow Digna to take action to preserve such Patent Rights included in the Joint Patent Rights at Digna's expense.

6.3.2 Enforcement. In the event that either Party identifies activities of Third Parties that (allegedly) infringe the Joint Patent Rights, it will notify the other Party and AMT shall decide whether it is necessary to commence proceedings as claimant and it shall be entitled

to request each and any of the CIMA Parties to join AMT as co-claimant, and the CIMA Parties shall decide whether to join or not at its sole election. If AMT succeeds in any such infringement proceedings whether at trial or by way of settlement, the first charge on any costs, damages or profits in such proceedings or settlement shall be the costs incurred by AMT and the CIMA Parties that have acted as co-claimants. If such sums are less than the costs incurred they shall be apportioned between AMT and the CIMA Parties in the proportion to the Parties' expenditure. Where the sums exceed the costs incurred, AMT on the one hand and the CIMA Parties that acted as co-claimant together on the other hand shall allocate the balance between them in the same proportion as the allocation of net revenues described in section 5.2. AMT is not entitled to settle such dispute without Digna's prior written consent. In case AMT decides not to commence proceedings as claimant, the CIMA Parties shall be entitled to commence them at its sole cost. In such a case, any sum derived from said infringement proceedings shall be for the CIMA Parties. The CIMA Parties shall not be entitled to settle such dispute without AMT's prior written consent.

6.3.3 Defense. In the event that one or more patents or patent applications included in the Joint Patent Rights are challenged by a Third Party (by way of interference, opposition, invalidity actions or otherwise), the Parties shall decide whether they want to jointly defend such patents or patent applications included in the Joint Patent Rights and jointly bear the costs. If the CIMA Parties do not want to bear half of the costs of such defense, AMT shall have the right (but not the obligation) to take the appropriate actions to defend such patents and patent application included in the Joint Patent Rights. In such event, AMT shall be entitled to deduct up to [\*\*]% of the costs incurred by AMT regarding such defense actions from the royalty payable by AMT to the CIMA Parties hereunder. Any awards will be allocated in accordance with Section 6.3.2. AMT (or the CIMA Parties, as the case may be) shall not be entitled to settle any challenge dispute without the other Party's prior written consent.

6.4 Cooperation. Each of the Parties shall make available to the other (or to the other's authorized attorneys, agents or representatives) its employees, agents or consultants to the extent necessary or appropriate to enable the appropriate Party to file, prosecute and maintain patent applications and resulting patents with respect to inventions owned by a Party, at the expense of this Party and for periods of time sufficient for such Party to obtain the assistance it needs from such personnel. Where appropriate, each of the Parties shall sign or cause to have signed all documents relating to said patent applications or patents at no charge to the other Party.

6.5 Third Party Claims of Infringement. If during the period of this Agreement, either Party receives any notice, claim or proceedings from any Third Party alleging infringement of that Third Party's intellectual property by reason of development or Commercialization of the Product, the Party receiving that notice shall (i) forthwith notify the other Party of the notice, claim or proceeding and (ii) neither Party shall make any admission of liability and notwithstanding that one of CIMA Parties may have received the notice, AMT shall at its own cost and expense be responsible for and shall have conduct of any and sole authority to defend or settle such claims or proceedings. If AMT reasonable believes that Third Party rights are valid and that infringement may be occurring, or believes that it is economically or otherwise advantageous to seek a license, it may, subject to Section 5.4, seek a license from such Third Party on appropriate commercial terms.

## **ARTICLE 7 ACCESS RIGHTS**

7.1 General. The Parties contemplate to collaborate on the identification and development of future products (other than the Products) within the Gene Therapy Field. Therefore, the Parties have agreed as follows:

7.1.1 Disclosure. During term of this License Agreement, the CIMA Parties shall disclose to AMT inventions such CIMA Party has, whether or not together with other CIMA Parties, conceived, developed or reduced to practice within the Gene Therapy Field ("Inventions"). Such inventions shall be disclosed in writing to AMT within reasonable time (no longer than [\*\*] months) after such invention has been conceived, developed or reduced to practice. The reports describing such inventions shall be deemed to be Confidential Information of the CIMA Parties.

7.1.2 The CIMA Parties herewith grant to AMT the exclusive first right to negotiate a license to Inventions and any IP associated therewith under market prevailing terms and conditions. The relevant CIMA Party shall be owner of any right, title and interest in such Invention.

7.1.3 In the event that AMT is interested in the further development and Commercialization of an Invention, AMT shall notify the CIMA Party that has sent the written report describing the Invention to AMT in writing within [\*\*] months after receipt of such report. In such event, the Parties shall in good faith negotiate the terms and conditions for an exclusive license to such Invention and any IP associated therewith. In case AMT has not notified in writing to the corresponding CIMA Party its interest in the Invention within the above [\*\*] months period, the CIMA Parties shall be entitled to offer such Invention to Third Parties (whether as a license, assignment or any other agreement), without any further right for AMT.

7.1.4 In case AMT has notified its interest within said [\*\*] months, but the Parties are not able to reach an agreement on the terms of such exclusive license for such Invention and IP rights associated therewith within [\*\*] months after the notification of its interest by AMT, the CIMA Parties may freely offer such Invention to Third Parties provided that they shall not accept an offer from a Third Party for such Invention unless they have first offered AMT the right to match such offer and AMT has not notified by writing within [\*\*] days its will to match such offer (and on the understanding that if AMT matches such offer, the CIMA Parties will grant an exclusive license under the Invention and IP rights associated therewith under the thus matched terms and conditions).

## ARTICLE 8 CONFIDENTIALITY

8.1 General. Each Party undertakes to keep strictly confidential the Confidential Information and to use it only for the purpose of this Agreement.

8.2 No disclosure / restriction to use. Each Receiving Party undertakes to not disclose the Confidential Information to any third party except to those of its officers and employees who need to have access to the same. Each Party shall before disclosing any Confidential Information to any of its officers or employees make each such person aware of such restrictions and to use and disclosure and shall procure that such persons comply with such restrictions. If the Receiving Party wishes to disclose Confidential Information to any consultant or advisor who is

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not an officer or employee it shall obtain the Disclosing Party's prior written consent and shall furnish to the Disclosing Party with a confidentiality agreement signed by such consultant or advisor on the same terms and conditions of this Agreement.

8.3 Exceptions. The obligations to maintain confidentiality and to respect the restriction of the use shall not apply where, as properly evidenced by documentation: (i) is or becomes patented, published or otherwise becomes publicly known other than by acts of the Party obligated not to disclose such Confidential Information in contravention of this Agreement; (ii) can be shown by written documents to have been disclosed to the Receiving Party by a Third Party, provided, that such information was not obtained by such Third Party directly or indirectly from the other Party under this Agreement; (iii) prior to disclosure under this Agreement, was already in the possession of the Receiving Party; or (iv) can be shown by written documents to have been independently developed by the Receiving Party without use of the other Party's Confidential Information or breach of any of the provisions of this Agreement.

8.4 Permitted disclosure. Notwithstanding the above obligations of confidentiality and non-use a Recipient Party may:

8.4.1 disclose Confidential Information to a Regulatory Authority as reasonably necessary to obtain Regulatory Approval in a particular jurisdiction to the extent consistent with the licenses granted under terms of this Agreement; and

8.4.2 disclose Confidential Information: (a) to the extent such disclosure is reasonably necessary to comply with the order of a court; or (b) to the extent such disclosure is required to comply with a legal requirement, including to the extent such disclosure is required in publicly filed financial statements or other public statements under rules governing a stock exchange (e.g. the rules of the Netherlands, United States Securities and Exchange Commission, NASDAQ, NYSE, UKLA or any other stock exchange on which securities issued by either Party may be listed); provided, to the extent possible bearing in mind such legal requirements and subject to the next subsequent sentence of this Section 8.4, such Party shall provide the other Party with a copy of the proposed text of such statements or disclosure [\*\*] Business Days in advance of the date on which the disclosure is to be made to enable the other Party to review and provide comments, which shall be followed as long as they are reasonable, unless a shorter review time is agreed. If the compliance with a legal requirements requires filing of this Agreement, the filing Party shall to the extent possible seek confidential treatment of portions of this Agreement from the relevant competent authority and shall provide the other Party with a copy of the proposed filings at least [\*\*] Business Days prior to filing for the other Party to review any such proposed filing. Each Party agrees that it will obtain its own legal advice with regard to its compliance with legal requirements and will not rely on any statements made by the other Party relating to such legal requirements

8.4.3 disclose Confidential Information by filing or prosecuting Patent Rights, the filing or prosecution of which is contemplated by this Agreement, without violating the above secrecy provision; it being understood that publication of such filings occurs in some jurisdictions within [\*\*] months of filing, and that such publication shall not violate the above secrecy provision;

8.4.4 disclose Confidential Information to such Recipient Party's Affiliates, contractors (including clinical researchers) distributors, licensee's, agents, consultants, as such

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Recipient Party reasonably determines is necessary to receive the benefit of this Agreement or to fulfill its obligations pursuant to this Agreement; provided, however, any such persons must be obligated to substantially the same extent as set forth in Section 8.2 to hold in confidence and not make use of such Confidential Information for any purpose other than those permitted by this Agreement;

8.4.5 disclose Confidential Information, only to the extent reasonably required (i) to its actual or potential investment bankers; (ii) to existing and potential investors in connection with an offering or placement of securities for purposes of obtaining financing for its business and to actual and prospective lenders for the purpose of obtaining financing for its business; and (iii) to a bona fide potential acquirer or merger partner for the purposes of evaluating entering into a merger or acquisition, provided, however, any such persons must be obligated to substantially the same extent as set forth in Section 8.2 to hold in confidence and not make use of such Confidential Information for any purpose other than those permitted by this Agreement; and

8.4.6 Disclose Confidential Information to its legal advisers for the purpose of seeking advice.

8.5 Press Release. Neither Party shall make any public announcement or statement to the public containing Confidential Information without the prior written consent of the other Parties. No such public announcement or statements shall be made without the prior review and consent of the appropriate individual designated for the purpose by the other Parties.

## ARTICLE 9 REPRESENTATIONS AND WARRANTIES

9.1 Mutual Warranties. Each Party represents and warrants to the other Parties that:

- (a) It has full power to extend the rights and licenses granted hereunder and perform its obligations hereunder;
- (b) It has full power and authority to enter into this Agreement and has taken all necessary action on its part required to authorize the execution and delivery of this Agreement;
- (c) The execution, delivery and performance of this Agreement and its compliance with the terms and provisions hereof does not conflict with, or result in a breach of any of the terms and provisions of, or constitute a default under any agreement to which any Party is a party; and
- (d) The execution, delivery and performance of this Agreement by each Party does not require the consent, approval or authorization of or notice, filing or registration with Regulatory Authority.

9.2 Warranties of CIMA Parties. The CIMA Parties jointly and severally represent and warrant to AMT that, at the Effective Date, (i) Proyecto is the owner of the undivided interest in the Joint Patent Rights; and that (ii) they have not previously entered into any agreement, whether written or oral, with respect to, or otherwise assigned, licensed, transferred,

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conveyed or otherwise encumbered its/their rights, title or interest in or to the Joint Patent Rights (including by granting any covenant not to sue with respect thereto).

9.3 Indemnification. AMT shall defend, indemnify and hold the CIMA Parties harmless from and against any Losses arising out of Third Party claims, suits or demands based on alleged or actual bodily injury or death or any other damage resulting from the development of Product or Commercialization of Product by AMT, its Affiliates or licensees. Losses shall not include any liability, claims, lawsuits, losses, damages, costs or expenses to the extent the same are determined to be the result of any CIMA Parties, their Affiliates, any Third Party engaged by Digna under the Collaborative Development Agreement and/or their directors officers, employees and agents negligence or willful misconduct.

AMT shall obtain and maintain insurance coverage in respect of the development and commercialization of the Product (notwithstanding Digna's obligation to obtain and maintain insurance coverage as set forth in the Collaborative Development Agreement). Evidence of the existence and continuation of such insurance shall be provided to the CIMA Parties and AMT, respectively, upon request.

9.4 The CIMA Parties shall immediately notify AMT (and each of the other CIMA Parties) in writing of any Third Party claim or action that may give rise to Losses (a "Claim Notice") for which AMT has to indemnify pursuant to Section 9.3. AMT undertakes at its expense, to assume sole control and responsibility for dealing with the Third Party and the Third Party claim, including the right to settle the Third Party claim on any terms AMT chooses, by giving written notice to Digna without [\*\*] days after receipt of a Claim Notice. The CIMA Parties shall be entitled to participate in, but not control, the defense of a Third Party claim by having their view regularly solicited by Digna who shall in turn liaise with AMT regarding the conduct of the Third Party Claim. Where proceedings are commenced, the CIMA Parties shall be entitled to retain counsel of their choice for such purpose, provided, however, that such retention shall be at each of the CIMA Parties' own cost and expense.

## ARTICLE 10 TERM / TERMINATION

10.1 This License Agreement shall commence on the Effective Date and shall continue until the payment obligations set out in Article 5 expire following which the Parties agree that the Agreement will have been fully performed. All licenses granted under this Agreement shall become perpetual, irrevocable, fully paid-up and royalty free on a country-by-country basis when there are not outstanding payment obligations in relation to such country.

10.2 Termination for breach or insolvency. Notwithstanding any other provision hereof, each Party may forthwith terminate this Agreement:

- (a) as a result of a material breach or default in the performance of any obligation, condition or covenant of this Agreement by the other Party or Parties, if such default or noncompliance shall not have been remedied within [\*\*] days after receipt by the defaulting Party of a notice thereof from the other Party; or
- (b) if the other Party receives suspension of payment or, whether voluntarily or involuntarily, is declared bankrupt, or if such Party becomes permanently unable

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to perform its obligations hereunder for reasons other than suspension of payment or bankruptcy, such as, for example, liquidation, dissolution or winding-up.

10.3 Termination for termination of the Collaborative Development Agreement. Each Party may terminate this Agreement in the event that the Collaborative Development Agreement is terminated.

10.4 Termination by AMT for convenience. AMT may terminate this Agreement for convenience upon 2 months written notice.

10.5 Effect of Termination.

10.5.1 In the event of termination, the licenses granted by the CIMA Parties to AMT as set forth in Article 3 of this Agreement shall terminate. In the event of termination by Digna pursuant to Section 10.2, by Digna or AMT pursuant to Section 10.3 (other than termination of the Collaborative Development Agreement by AMT for breach or insolvency of Digna) or Section 10.4, the CIMA Parties shall have the exclusive rights, with the right to grant sublicenses, to use the Joint Patent Rights for the further development and Commercialization of Products as treatment or prevention of the Disorder without financial obligations to AMT, and each of the CIMA Parties collectively on the one hand and AMT on the other hand shall have the non-exclusive rights, without



the right to sublicense, to use the Joint Patent Rights for the development and Commercialization of products as treatment or preventions of disorders other than the Disorder without financial obligations to the other Party or Parties. In the event of termination by AMT pursuant to Section 10.2 or by Digna or AMT pursuant to Section 10.3 (other than termination of the Collaborative Development Agreement by Digna for breach or insolvency of AMT), AMT shall have the exclusive rights, with the right to grant sublicenses, to use the Joint Patent Rights for the further development and Commercialization of Products or products as treatment or prevention of the Disorder, or disorders other than the Disorder without financial obligations to CIMA Parties.

10.6 The Articles 2, 3.1, 5.7, 6, 8, 9, 12, 13 and 14 survive expiration or termination of this Agreement for any reason (subject to Section 10.5).

## ARTICLE 11 ASSIGNMENT

Save as otherwise provided in this Agreement no Party shall without the prior written consent of the other assign the benefit and/or burden of this Agreement provided always that any Party may assign this Agreement to an Affiliate of said Party or any purchaser of the whole or part of said Party's assets or to a company with which said Party is merging, provided that such Affiliate, purchaser or merger company undertakes to the other Parties to be bound by the terms of this Agreement. The CIMA Parties shall not transfer, assign or encumber (their interest in the) ownership of the CIMA Background IP or the Joint Patent Rights without AMT's prior written consent, other than to an Affiliate or in connection with an above permitted assignment of its rights. AMT shall not transfer, assign or encumber its interest in the ownership of the Joint Patent Rights without Digna's prior written consent other than to an Affiliate or in connection with an above permitted assignment of its rights.

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## ARTICLE 12 NOTICES

Any notices required or provided under this Agreement shall be in writing and shall be given by facsimile or by certified mail addressed to the applicable Party as set out below:

<b>AMT</b>	Mr. Piers Morgan PO Box 22506 1100DA Amsterdam The Netherlands
<b>UTE CIMA</b>	Mr. Antonio Martin Cantón UTE PROYECTO CIMA AVDA. PÍO XII, 22, OFICINA 1. 31008 PAMPLONA, NAVARRA Spain
<b>FIMA</b>	Mr. Franciso Errasti Goenaga Calle Pintor Paret 5,1° F Pamplona, Spain
<b>DIGNA</b>	Mr. Pablo Ortiz Betes DIGNA BIOTECH S.L.AVDA. PÍO XII, 22, OFICINA 2. 31008 PAMPLONA, NAVARRA Spain
<b>PROYECTO</b>	Mr. Antonio Martin Cantón PROYECTO DE BIOMEDICINA CIMA AVDA. PÍO XII, 22, OFICINA I. 31008 PAMPLONA, NAVARRA Spain

## ARTICLE 13 GOVERNING LAW

The validity construction and performance of this Agreement shall be governed by the laws of The Netherlands.

## ARTICLE 14 JURISDICTION

All disputes arising out of or in connection with this Agreement shall be finally settled under the Rules of Arbitration of the International Chamber of Commerce by one or more arbitrators appointed in accordance with said Rules. The arbitration to take place in Paris and to be conducted in English.

## ARTICLE 15 MISCELLANEOUS

### 15.1 Interpretation.

15.1.1 If an ambiguity or a question of intent or interpretation arises with respect to this Agreement, this Agreement shall be construed as if drafted jointly by the Parties and no presumption or burden of proof shall arise favoring or disfavoring any Party by virtue of the authorship of any provisions of this Agreement.

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15.1.2 Whenever the context may require, any pronoun shall include the corresponding masculine, feminine and neuter forms. The words "include", "includes" and "including" shall be deemed to be followed by the phrase "without limitation." The word "will" shall be construed to have the same meaning and effect as the word "shall." Unless the context requires otherwise, (A) any definition of or reference to any agreement, instrument or other document herein shall be construed as referring to such agreement, instrument or other document as from time to time amended, supplemented or otherwise modified (subject to any restrictions on such amendments, supplements or modifications set forth herein or therein), (B) any reference to any laws herein shall be construed as referring to such laws as from time to time enacted, repealed or amended, (C) any reference herein to any Person shall be construed to include the Person's successors and assigns, (D) the words "herein", "hereof and "hereunder", and words of similar import, shall be construed to refer to this Agreement in its entirety

and not to any particular provision hereof, (E) any reference herein to the words “mutually agree” or “mutual written agreement” shall not impose any obligation on either Party to agree to any terms relating thereto or to engage in discussions relating to such terms except as such Party may determine in such Party’s sole discretion and; (F) all references herein to Articles, Sections or Schedules shall be construed to refer to Articles, Sections and Schedules of this Agreement.

15.2 Force Majeure. Neither Party shall be held liable or responsible to the other Party nor be deemed to have defaulted under or breached this Agreement for failure or delay in fulfilling or performing any term of this Agreement when such failure or delay is caused by or results from causes beyond the reasonable control of the affected Party, including fire, floods, embargoes, war, acts of war (whether war is declared or not), insurrections, riots, civil commotions, strikes, lockouts or other labor disturbances, acts of God or acts, omissions or delays in acting by any governmental authority or the other Party; *provided, however,* that the Party so affected shall use Commercially Reasonable Efforts to avoid or remove such causes of non-performance, and shall continue performance hereunder with reasonable dispatch wherever such causes are removed. Each Party shall provide the other Parties with prompt written notice of any delay or failure to perform that occurs by reason of force majeure. The Parties shall mutually seek a resolution of the delay or the failure to perform in good faith.

15.3 Severability. Each Party hereby agrees that it does not intend to violate any public policy, statutory or common laws, rules, regulations, treaty or decision of any government agency or executive body thereof of any country or community or association of countries. Should one or more provisions of this Agreement be or become invalid, the Parties hereto shall substitute, by mutual consent, valid provisions for such invalid provisions which valid provisions in their economic effect are sufficiently similar to the invalid provisions that it can be reasonably assumed that the Parties would have entered into this Agreement with such valid provisions. In case such valid provisions cannot be agreed upon, the invalidity of one or several provisions of this Agreement shall not affect the validity of this Agreement as a whole, unless the invalid provisions are of such essential importance to this Agreement that it is to be reasonably assumed that the Parties would not have entered into this Agreement without the invalid provisions.

15.4 Exhibits. The Exhibits to this Agreement form an integral part of this Agreement.

15.5 Entire Agreement. This Agreement (including the Exhibits thereto) contains the entire understanding of the Parties with respect to the subject matter hereof and supersedes all

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prior or contemporaneous oral or written agreements of the Parties with respect to the subject matter hereof, including the Previous Agreements. This Agreement may be amended, or any term hereof modified, only by a written instrument duly executed by both Parties hereto.

15.6 Headings. The captions to the several Articles and Sections hereof are not a part of this Agreement, but are merely guides or labels to assist in locating and reading the several Articles and Sections hereof.

15.7 Independent Contractors. It is expressly agreed that the Parties to this Agreement shall be independent contractors and that the relationship between the Parties shall not constitute a partnership, joint venture or agency. Neither Party shall have the authority to make any statements, representations or commitments of any kind, or to take any action, which shall be binding on the other(s), without the prior consent of the other Parties to do so.

15.8 Waiver. Except as expressly provided herein, the waiver by either Party hereto of any right hereunder or of any failure to perform or any breach by the other Party shall not be deemed a waiver of any other right hereunder or of any other failure to perform or breach by said other Party, whether of a similar nature or otherwise, nor shall any singular or partial exercise of such right preclude any further exercise thereof or the exercise of any other such right.

15.9 Counterparts. This Agreement may be executed in one more counterparts, each of which shall be deemed an original, but all of which together shall constitute one and the same instrument.

15.10 Benefit. Nothing in this Agreement or the agreements referred to herein, expressed or implied, shall confer on any person other than the Parties hereto or thereto, or their respective permitted successors or assigns, any rights remedies, obligations or liabilities under or by reason of this Agreement, the agreements referred to herein, or the transactions contemplated herein or therein.

15.11 Further Assurances. Each Party shall, as and when requested by another Party, do all acts and execute all documents as may be reasonably necessary to give effect to the provisions of this Agreement.

[Remainder of page intentionally left blank]

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IN WITNESS WHEREOF, the Parties have executed this Collaboration Agreement as of the date first written above.

\_\_\_\_\_  
FIMA  
By: Mr. Franciso Errasti Goenaga  
Title: Presidente

\_\_\_\_\_  
/s/ Antonio Martin Cantón  
UTE CIMA  
By: Antonio Martin Cantón  
Title: Gerente – Manager

\_\_\_\_\_  
/s/ Mr. Antonio Martin Cantón  
Proyecto de Biomedicina CIMA S.L.  
By: Mr. Antonio Martin Cantón  
Title: Director General – General Manager

/s/ Pablo Ortiz Betés  
Digna Biotech S.L.  
By: Mr. Pablo Ortiz Betés  
Title: Director General

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/s/ Piers Morgan  
Amsterdam Molecular Therapeutics (AMT) B.V.  
By: Mr. Piers Morgan  
Title: Chief Financial Officer

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## Exhibit 1

JOINT PATENT RIGHTS

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## Exhibit 2

COLLABORATIVE DEVELOPMENT AGREEMENT BETWEEN DIGNA AND AMT

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### COLLABORATIVE DEVELOPMENT AGREEMENT

by and among

DIGNA BIOTECH, S.L.

AND

AMSTERDAM MOLECULAR THERAPEUTICS (AMT) B.V.

dated as of 21<sup>st</sup> of May, 2010

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### COLLABORATIVE DEVELOPMENT AGREEMENT

THIS COLLABORATIVE DEVELOPMENT AGREEMENT dated as of 21 May, 2010 (the “Agreement”) is made by and among:

- (1) Digna Biotech, S.L. (“**Digna**”) with corporate address at C/ Etxesakan 28, oficina 5, 31180 Cizur Maryo, Navarra, Spain, beare of Tax Identification Number B-31778509, duly represented by Mr. Pablo Ortiz Betés.  
  
and
- (2) Amsterdam Molecular Therapeutics (AMT) B.V. (“**AMT**”) a company with limited liability incorporated under the laws of The Netherlands with registered office at Meibergdreef 61, NL-1105 BA Amsterdam, The Netherlands, duly represented by Mr. Piers Morgan

## WHEREAS

- (A) Digna and AMT, together with FIMA, UTE CIMA and Proyecto (as defined in the License Agreement), have entered into a License Agreement of even date herewith;
- (B) Digna has broad expertise and Know How in the field of acute intermittent porphyria and has, through hospitals with which it collaborates, access to relevant patient populations;
- (C) AMT has broad expertise and Know How in the field of development and manufacturing of products as gene therapy treatment;
- (D) the Parties now want to combine their expertise and knowledge aimed at the preclinical and clinical development of a gene therapy product as treatment for and/or prevention of acute intermittent porphyria under the terms and conditions set forth below;

IT IS NOW AGREED AS FOLLOWS

For purposes of this Agreement, the terms defined in this Article 1 shall have the meanings specified below. Certain other capitalized terms are defined elsewhere in this Agreement.

1.1 “Affiliate” any company, partnership or other business entity which Controls, is Controlled by or it under common Control with any of the Parties. For the purposes of this definition “Control” refers to any of the following (i) the possession, directly or indirectly, of the power to direct the management or policies of an entity, whether through ownership of voting securities, by contract or otherwise; (ii) ownership of more than fifty percent (50%) of the voting securities entitled to vote for the election of directors in the case of a corporation, or of more than fifty percent (50%) of the equity interest in the case of any other type of legal entity; (iii) status as a general partner in any partnership, or any other arrangement whereby a Party controls or has

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the right to control the board of directors or equivalent governing body of a corporation or other entity.

1.2 “Agreement” means this Collaborative Development Agreement.

1.3 “AMT Background IP” has the same meaning as assigned to it in the License Agreement.

1.4 “CIMA Background IP” has the same meaning as assigned to it in the License Agreement.

1.5 “CIMA Parties” has the meaning assigned to it in the License Agreement.

1.6 “Clinical Trial” means each and any clinical trial and/or other study where the Product is administered to humans or that involves human subjects carried out in the context of the current Agreement, as set out in the Development Plan and Protocol. Specifically, Clinical Trial refers to Phase I/II clinical trial. Any other clinical trial not set out in the Development Plan but added to the Development Plan by decision of the Joint Steering Committee will be covered by a separate Agreement. .

1.7 “Confidential Information” means, subject to the exceptions set forth in Article 10.3 (i) the terms and conditions of this Agreement, for which each Party will be considered a Disclosing Party and a Recipient Party; and (ii) any non-public information, whether or not patentable, disclosed or provided by one Party to the other Party in connection with this Agreement, including, without limitation, any information which release is likely to prejudice the commercial interests of the parties, or is considered as a trade secret, including information regarding such Party’s strategy, business plans, objectives, research, technology, products, business affairs or finances including any non-public data relating to development or Commercialization of any Product and other information of the type that is customarily considered to be confidential information by parties engaged in activities that are substantially similar to the activities being engaged in by the Parties under this Agreement, for which the Party making such disclosure will be considered the Disclosing Party and the receiver will be the Recipient Party.

1.8 “Development Plan” means the comprehensive plan (including activities assigned to each of Digna and AMT, timelines and budget) for the preclinical and clinical development of the Product aimed at obtaining Regulatory Approval for the Product, attached hereto as Exhibit 1.

1.9 “Disclosing Party” means the Party which discloses Confidential Information to the other Party or Parties.

1.10 “Disorder” means acute intermittent porphyria

1.11 “Documents” means analyses, books, CD-ROM, USB stick, charts, comments, computations, designs, discs, diskettes, files, graphs, ledgers, notebooks, paper, photographs, plans, records, recordings, reports, research notes, tapes and other graphic or written data or other media and other computer information storage means on which Know How is permanently

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stored and advertising and promotional materials of any nature whatsoever including preparatory materials for the same.

1.12 “Effective Date” means the date first set forth above.

1.13 “EU Clinical Trial Directive” means Directive 2001/20/EC of the European Parliament and of the Council of 4 April 2001 on the approximation of the laws, regulations and administrative provisions of the Member States relating to the implementation of good clinical practice in the conduct of clinical trials on medicinal products for human use” and all legislation of the EU member states implementing this directive.

1.14 “GCP” means the ICH Harmonized Tripartite Guideline for Good Clinical Practice together with such other good clinical practice requirements as are specified in Directive 2001/20/EC of the European Parliament and the Council of 4 April 2001 relating to medicinal products for human use and in guidance published by the European Commission pursuant to such Directive and including the EU detailed guidelines on good clinical practice specific to advanced therapy medicinal products of December 3, 2009.

1.15 “Hospital” means Clínica Universidad de Navarra and any other premises as designated by the Joint Steering Committee to conduct the Clinical Trial.

1.16 “IMPD” means Investigational Medicinal Product Dossier as defined in Clinical Trials Directive (2001/20/EC).

1.17 “Intellectual Property” or “IP” means Patent Rights, Know How and Materials together.

1.18 “Joint Steering Committee” means the committee to be established by the Parties to manage the Development Program pursuant to Section 7.1.

1.19 “Know-How” means technical and other information which is not in the public domain, including information comprising or relating to concepts, discoveries, data, designs, formulae, ideas, inventions, Materials, methods, models, research plans, procedures, designs for experiments and tests and results of experimentation and testing (including results of research or development) processes (including manufacturing processes, specifications and techniques),

laboratory records, chemical, pharmacological, toxicological, clinical, analytical and quality control data, clinical and non-clinical trial data, case report forms, data analyses, reports, manufacturing data or summaries and information contained in submissions to and information from ethical committees and Regulatory Authorities. Know How includes Documents containing Know How, including but not limited to any rights including trade secrets, copyright, database or design rights protecting such Know How. The fact that an item is known to the public shall not be taken to preclude the possibility that a compilation including the item, and/or a development relating to the item, is not known to the public.

1.20 “License Agreement” means the license agreement of even date herewith between Digna, AMT, UTE CIMA, FIMA and Proyecto.

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1.21 “Losses” means any and all losses, damages, liabilities, costs and expenses (including, without limitation, reasonable attorneys’ fees and expenses). In calculating “Losses”, the duty to reasonably mitigate on the part of the Party suffering the Losses shall be taken into account.

1.22 “Marketing Authorisation Application” or “MAA” means a new drug license application filed with the competent European Regulatory Authorities to obtain Regulatory Approval for a pharmaceutical product in Europe, or any equivalent application filed with the Regulatory Authority in or for a country or group of countries to obtain Regulatory Approval for a pharmaceutical product in or for that country or with that group of countries.

1.23 “Materials” means any chemical or biological substances including but not limited to blood samples, nucleotide or nucleotide sequence including DNA and RNA sequences, genes, vector or construct including plasmids, phages or viruses, host organism including bacteria, fungi, algae, protozoa and hybridoma’s, eukaryotic or prokaryotic cell line or expression system or any development strain or product of that cell line or expression system, protein including any peptide or amino acid sequence, enzyme, antibody or protein conferring targeting properties and any fragment of a protein or a peptide enzyme or antibody, assay or reagent, any other genetic or biological material or micro-organism.

1.24 “Monitor” means one or more persons appointed by Digna to monitor compliance of the Clinical Trials with GCP and to conduct source data verification.

1.25 “Observational Study” means a Clinical Trial aimed at assessing baseline parameters of patients with the Disorder as further described in the Development Plan.

1.26 “Parties” means the parties to this Agreement and “Party” means a party to this Agreement.

1.27 “Patent Rights” means a (i) all national, regional and international patents and patent applications, including provisional patent applications, (ii) all patent applications filed either from such patents, patent applications or provisional applications or from an application claiming priority from any of these, including divisional, continuations, continuations-in-part, provisions, converted provisionals and continued prosecution applications, (iii) any and all patents that have issued or in the future issue from the foregoing patent applications (i) and (ii), including author certificates, inventor certificates, utility models, petty patents and design patents and certificates of invention, (iv) any and all extensions or restorations by existing or future extension or restoration mechanisms, including revalidations, reissues, re-examinations and extensions (including any supplementary protection certificates and the like) of the foregoing patents or patent applications (i), (ii) and (iii), and (v) any similar rights, including so-called pipeline protection, or any importation, revalidation, confirmation or introduction patent or registration patent or patent of additions to any such foregoing patent applications and patents.

1.28 “Phase I/II” means, for the purpose of this Agreement, a Clinical Trial conducted by a Qualified Service Provider aimed at preliminary determination of safety in patients affected by the Disorder and (ii) determination of dose ranges and a preliminary determination of efficacy in patients affected by the Disorder as further described in the Development Plan.

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1.29 “Phase II/ III” means pivotal human clinical trials conducted at multiple sites, which are sufficiently powered and designed to establish safety and efficacy of one or more particular doses in patients being studied and to provide the statistical basis for marketing approval for the respective drug (for example, as described in 21 C.F.R. § 312.21, or similar clinical study legislation or guidelines in a country other than the United States).

1.30 “Principal Investigator” means Prof. Jesus Prieto or any other person agreed by the Parties to replace him.

1.31 “Product” means a AAV vector containing porphobilinogen deaminase (PBGD) manufactured by AMT or its Affiliates for use within the scope of the Development Plan.

1.32 “Protocol” means the description of a Clinical Trial to be conducted under the Development Plan and all amendments thereto as the Joint Steering Committee may from time to time agree. The Protocol, once determined by the Joint Steering Committee, will be attached to this Agreement as **Exhibit 2**. Any amendments will be signed by the Parties and form a part of this Agreement.

1.33 “Qualified Service Providers” means Third Parties engaged by Digna in the execution of its preclinical or clinical activities under the Development Plan, including CIMA, the Hospital and the Principal Investigator, and that (i) are discussed in the meeting of the Joint Steering Committee prior to its engagement and to which AMT has not imposed reasonable and substantiated objections (ii) meet the quality and other criteria imposed by Spanish legislation regarding Clinical Trial and/or the Spanish Regulatory Authorities for the execution of such activities and (iii) are, if other than CIMA, the Hospital and/or the Principal Investigator, under a written obligation between Digna and the Qualified Service Provider to comply with the obligations of Digna set forth in this Agreement regarding confidentiality, publication and ownership of Results as if they were a Party thereto or, if relating to the conduct of a Clinical Trial, have entered into a written agreement with Digna.

1.34 “Receiving Party” means any Party receiving Confidential Information from another Party;

1.35 “Regulatory Approval” means all approvals from Regulatory Authorities in any country in the Territory required lawfully to develop, clinically test, manufacture and market the Product in any such country, any establishment license application filed with the FDA or other Regulatory Authority to obtain approval of the facilities and equipment to be used to manufacture a Product, any Investigational New Drug or other investigational filing, including but not limited to any authorization for the import, manufacture and clinical testing of the Product (whether or not in filled and finished form), and any product pricing approvals where applicable.

1.36 “Regulatory Authority” means any relevant national (e.g., the FDA, EU member states authorities), supra-national (e.g., the European Commission, the Council of the European Union, or the EMEA), or other relevant governmental entity in any jurisdiction of the world involved in the granting of Regulatory Approvals for pharmaceutical product.

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1.37 “Results” means data, Know How, Materials, inventions, Patent Rights and all other information resulting from the activities by Digna or its Qualified Service Providers under this Agreement, excluding the Joint Patent Rights (as defined in the License Agreement), excluding any IP that has been developed prior to the Effective Date under the collaborative research programs jointly carried out by the Parties, excluding the AMT Background IP and excluding the CIMA Background IP.

1.38 “Territory” means the world.

1.39 “Third Party” means a party other than any of the Parties or any of their respective Affiliates.

## ARTICLE 2 DEVELOPMENT PLAN GENERAL

2.1 Development Plan. The activities assigned to each of Digna and AMT with regard to the preclinical and (once successful) clinical development of the Product are set forth in the Development Plan attached hereto as **Exhibit 1**. The Development Plan sets forth in detail (i) the proposed overall program of development for the Product, including pre-clinical studies, toxicology, formulation, manufacturing, Clinical Trials and regulatory plans up to and including Phase I/II Clinical Trials, (ii) a summary of estimated costs expected to be incurred by each Party hereunder in performing its activities under the Development Plan and (iii) the timelines. In general terms (i) AMT shall be responsible for the production of the Product for preclinical studies and Clinical Trials in accordance with all local and European legislation and the Good manufacturing practice (GMP) Guidelines., (ii) Digna shall be responsible for the execution of the preclinical studies and the drafting of the Protocol(s) and (iii) Digna shall act as Sponsor of the Clinical Trial.

2.2 Updated Development Plan. The Joint Steering Committee may decide to update the Development Program at a later date to revise or expand the Development Program, *provided, however*, that the Joint Steering Committee shall not assign additional activities to Digna unless (i) AMT agrees to increase its funding for the Development Plan accordingly or (ii) such additional activities are covered under a government grant or subsidy.

2.3 Qualified Service Providers. Digna may not subcontract its activities under the Development Plan to Third Parties unless such Third Party qualifies as a Qualified Service Provider as defined herein.

2.4 Execution of Development Plan. Digna and AMT shall use reasonable efforts to execute and substantially perform (or have performed by Qualified Service Providers) the activities assigned to each Party under the Development Program in accordance with the budget and timelines set forth in the Development Plan.

## ARTICLE 3 CLINICAL TRIAL

3.1 General. Digna shall act as the Sponsor of the Clinical Trial to be conducted by the Hospital and the Principal Investigator.

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3.2 Separate Clinical Trial Agreement. Digna shall enter into a separate Clinical Trial agreement with the Hospital, the Principal Investigator and each Qualified Service Provider under terms and conditions that are customary for the kind of services to be provided with regard to human clinical trials, wherein Digna shall impose on the Hospital, the Principal Investigator and other Qualified Service Providers the obligations as set forth in this Article 3, 4.3, 5.1, 9, 10 and 11 and wherein AMT shall be named as third party beneficiary.. AMT shall have the right to review such draft agreements before they are entered into to verify that the obligations of Hospital, Principal Investigator and other Qualified Service Providers as reflected in this Article 3, 4.3, 5.1, 9, 10 and 11 are properly included.

3.3 Trial Site. The Phase I/II Clinical Trial shall be conducted at the site of the Hospital under supervision of the Principal Investigator. AMT may, however, require Digna to add additional Hospitals for the conduct of the Clinical Trials pursuant to Section 3.9 hereof.

3.4 Obligations Digna. Digna shall use reasonable efforts to procure compliance of the Hospital and the Principal Investigator conducting the Clinical Trial with all obligations imposed on Digna under this Agreement to the extent they relate to the conduct of the Clinical Trial as well as the obligations regarding confidentiality, publications and ownership of Results.

3.5 Obligations AMT: AMT shall share with Digna its expertise and experience in the field of conducting clinical trials with gene therapy products and shall assist Digna in the preparation of the investigator’s brochure, whereby Digna, however, acknowledges that Digna is, as sponsor of the Clinical Trials, responsible for the proper conduct thereof. AMT shall be responsible for including in the IMPD all required information regarding the manufacture of the Product. .

3.6 Protocol. Digna shall be in the lead as to the design of the Protocol for the Clinical Trial, provided that (i) the Protocol is in accordance with the Development Plan and (ii) furthermore provided that any Protocol and any deviation to the Protocol (whether or not instigated by the Regulatory Authorities in Spain) will be agreed with AMT before (re)submitting. Digna will make its best efforts to ensure that the Hospital and the Principal Investigator shall conduct the Clinical Trial in accordance with: (i) the Protocol (once established by the Joint Steering Committee) for such Clinical Trial; (ii) the terms and conditions of the approval of the relevant ethics committee and (iii) instruction by AMT as to the handling and use of the Product. The Principal Investigator and the Hospital shall not consent to any change in the Protocol requested by a relevant ethics committee without the prior written consent of Digna and AMT.

3.7 Medical Ethical Approval. Digna will make its best efforts to ensure that the Hospital and Principal Investigator shall not administer Product to any Clinical Trial subject and that no other clinical intervention mandated by the Protocol takes place in relation to any such Clinical Trial subject until it is satisfied that all relevant regulatory and ethics committee approvals have been obtained.

3.8 Regulatory and GCP Compliance. Digna shall comply with all laws and regulations applicable to the performance of the Clinical Trial including, but not limited to the GCP, the World Medical Association Declaration of Helsinki and any and all local applicable

laws and regulations, as amended from time to time including but not limited to, where applicable, Spanish regulations regarding gene therapy, informed consent and privacy regulations and shall use reasonable efforts to ensure that the Hospital, the Principal Investigator and any other Qualified Service Provider will comply with such laws and regulations as well..

3.9 Use of Product. Digna shall use reasonable efforts to ensure that Hospital and Principal Investigator will not use the Product for any purpose other than the conduct of the Clinical Trial and upon termination or expiration of this Agreement all unused Product shall, at AMT's option, either be returned to AMT or disposed of in accordance with the Protocol.

3.10 Recruitment. Digna shall oblige the Hospital and Principal Investigator to use their best efforts to recruit the number of Clinical Trial subjects as set forth in the Development Plan (or, if deviating, the Protocol for such Clinical Trial) and shall conduct the Clinical Trial in accordance with the timelines set forth in the Development Plan and the Protocol. If, for reasons other than AMT's breach of its obligations under this Agreement, recruitment of Clinical Trial subjects for the Phase I/II Clinical Trial is proceeding at a rate below that, at the discretion of the Joint Steering Committee, required to enable the relevant timeline in the Development Plan to be met, AMT may by notice to Digna require recruitment by the Hospital to cease and/or to add additional hospitals as designed by the Joint Steering Committee, for the conduct of such Clinical Trial. The terms of the Agreement shall thereafter relate to the number of Clinical Trial subjects who have been accepted for treatment in the Clinical Trial by the Hospital at the date of such notice and the funding as set forth in Article 8 hereof, to the extent it relates to such Clinical Trial, shall be allocated by Digna between the Hospital and such other hospitals involved in such Clinical Trial.

3.11 Monitor. Digna ensures that the Hospital and Principal Investigator shall permit the Monitor access to the records of Clinical Trial subjects for monitoring and source data verification, such access to be arranged at mutually convenient times and on reasonable notice. Each of Digna and AMT will alert the Hospital and Principal Investigator promptly to significant issues (in the opinion of the Monitor) relating to the conduct of the Clinical Trial. In the event that AMT reasonably believes there has been any research misconduct in relation to the Clinical Trial, Digna shall use reasonable efforts to procure that the Hospital and Principal Investigator shall provide all reasonable assistance to any investigation into any alleged research misconduct undertaken by or on behalf of AMT. At its conclusion, AMT, Digna, the Hospital and Principal Investigator shall review the conduct of the Clinical Trial at the trial site set forth in Section 3.3 hereof, such review to take place within [\*\*] months of trial site close-out. Digna shall send copies of the reports of the Monitor to the members of the Joint Steering Committee.

3.12 Samples. Digna shall use reasonable efforts to procure that Hospital and Principal Investigator shall test any clinical samples required to be tested during the course of the Clinical Trial in accordance with the Protocol and at a laboratory that qualifies as a Qualified Service Provider.

3.13 Follow-up. Digna shall use reasonable efforts to provide that Hospital and Principal Investigator shall give follow-up to subjects for at least the time that is required under the relevant laws and regulations pertaining to (preclinical and clinical trials with) gene therapy products.

3.14 Not debarred. Digna shall use its best efforts to ensure that neither the Investigator nor any other person involved in the conduct of the Clinical Trials is or has been debarred.

3.15 No adverse activities. Digna shall use reasonable efforts to procure that neither the Hospital nor the Principal Investigator shall during the term of this Agreement conduct any human clinical trial which might adversely affect the Hospital's or Principal Investigator's ability to perform its obligations under this Agreement within the timelines set forth in the Development Plan. Digna, furthermore, will negotiate in order to procure a commitment by both the Hospital and the Principal Investigator in the sense that they shall not be engaged in human clinical trials involving gene therapy and/or enzyme replacement therapy and/or any product aimed at treatment or prevention of the Disorder, during the term of this Agreement.

#### ARTICLE 4 REGULATORY FILINGS

4.1 Filing of the IMPD. Digna shall file an IMPD for the Product with the Regulatory Authority in Spain. Without prejudice to the fact that Digna will act as sponsor of the Clinical Trials AMT shall be responsible for the IMPD to the extent it relates to the Product and the manufacturing thereof. Digna acknowledges that AMT's information regarding the manufacture of the Product is highly sensitive and proprietary and that AMT will, to the extent allowed by the Regulatory Authorities in Spain, provide such information to the Regulatory Authority in Spain without allowing Digna access thereto. Digna shall be responsible for the IMPD to the extent it relates to the information relating the Disorder and the Clinical Trials. For the avoidance of doubt: AMT shall be the sole Party entitled to use, develop and commercialize the Product and to file any additional Regulatory filings for the Product, including an MAA. To the extent required, Digna herewith assigns in advance (and shall, if such assignment in advance is not possible, upon first request of AMT assign), any and all of its rights that may be obtained by Digna by virtue of filing the IMPD, to AMT.

4.2 Other regulatory interactions. Digna shall, to the extent permitted by the Regulatory Authorities in Spain, act to communicate with the Regulatory Authority in Spain with regard to the Clinical Trials and the IMPD save for the part of the IMPD that relates to the Product and the manufacturing of the Product. AMT shall act to communicate with the Regulatory Authorities with regard to the Product and the manufacturing of the Product. No party will approach, consult and/or negotiate with regulatory authorities without an expressed consent from the other party. Any regulatory authority interaction will be jointly prepared and agreed between Digna and AMT in advance of any such interactions (without prejudice to AMT's legitimate interest to keep the information regarding the manufacture of the Product confidential and to minimize access by Digna thereto to the extent allowed by the relevant laws and regulations.

4.3 Local ethical committee approvals. Ethics Committee. Digna shall use reasonable efforts to ensure that the Hospital and the Principal Investigator shall collaborate for obtaining and maintaining all approvals from the relevant local research ethics committee of the Hospital (so other than the IMPD) for the conduct of the Clinical Trial and that the Hospital and the Principal Investigator shall keep Digna and AMT fully apprised of the progress of ethics

committee submissions and that they shall provide Digna with all correspondence relating to such submissions.

## ARTICLE 5 AUDIT

5.1 Audit Rights. AMT shall be entitled to have reasonable access to the facilities of Digna and its Qualified Service Providers (including the Hospital) during regular business hours with reasonable frequency (which shall not be in any case more than [\*\*] per facility per organization) and upon reasonable advance notice and, with regard to each Qualified Service Provider, prior to the execution of the agreement between Digna and such Qualified Service Provider (pre-audit), at AMT's own expense and during the term of this Agreement, to records and facilities of Digna and its Qualified Service Providers (including the Hospital) relating to the Development Program, but only to the extent reasonably necessary for AMT to ensure compliance by Digna and its Qualified Service Providers with the Development Plan, the Protocol, GCP and other applicable laws and regulations. In no event shall personal data (as defined in the Spanish Data Protection Act), be disclosed to AMT.

## ARTICLE 6 MANUFACTURING

6.1 Manufacturing and Supply Product. AMT shall be solely responsible for the manufacture and supply of the Product for use in the activities under the Development Plan. To the extent Product is to be used for Clinical Trial, AMT shall supply Product that meets the requirements of the Regulatory Authorities that are competent to obtain Regulatory Approval for the IMPD referred to in Section 4.1. AMT may, in its sole discretion, subcontract with Third Parties for the manufacture, supply or packaging of the Product. AMT shall not supply the Product directly to the Hospital without the prior written instruction of Digna to do so.

6.2 AMT will be solely responsible for obtaining and maintaining all the necessary authorizations or clearances set forth in the national and supra-national legislation regarding the manufacturing of the Investigational Medicinal Product and shall provide Digna (or the Hospital directly) with sufficient quantities of Product to execute the preclinical work and/or the Clinical Trials, free of charge.

6.3 AMT shall, upon request of Digna, provide reasonable assistance to Digna in order to comply with all the relevant obligations that are required in its condition of Sponsor as stated in the enforceable legislation, the regulation of clinical good practices or the obligations which may be required at any time by the Regulatory Authority and, if were the case, by the Hospital or the Principal Investigator provided, however, that Digna (and not AMT) shall be responsible that the Clinical Trials and other activities in the course of the development of the Product (other than the manufacture of the Product) is in compliance with applicable laws and regulations.

6.4 DIGNA will have the right to reject the Product if it does not meet the quality, safety and stability specifications previously agreed by the Joint Steering Committee.

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6.5 The Product will be transported by AMT (at AMT's expense and under AMT's responsibility) to the clinical site of the Hospital referred to in Section 3.3 hereof or at such other place as Digna and AMT may jointly decide.

## ARTICLE 7 JOINT STEERING COMMITTEE

### 7.1 Joint Steering Committee.

7.1.1 Formation and Composition. Forthwith upon signing this Agreement, Digna and AMT will establish a committee to oversee and manage the activities pursuant to this Agreement (the "Joint Steering Committee"). The Steering Committee shall be composed of [\*\*] representatives appointed by Digna and [\*\*] representatives appointed by AMT, *provided* that the size of the Joint Steering Committee may be increased by unanimous agreement of the Joint Steering Committee members so long as the membership has equal representation by Digna and AMT. The initial representatives on the Joint Steering Committee of Digna shall be [\*\*], and AMT's initial representatives on the Joint Steering Committee shall be [\*\*] shall designate one (1) of its representatives on the Joint Steering Committee to act as Chair, and the Joint Steering Committee shall appoint one (1) of its members to act as Secretary. Members of the Joint Steering Committee may be represented at any meeting by another member of the Joint Steering Committee or by a deputy. Each Party may change one or more of its representatives to the Joint Steering Committee at any time, *provided* that any such representatives will be senior officers and/or managers of the respective Party, its Affiliates, divisions or business units. Additionally, each of the Parties may appoint a non-voting special advisor to the Joint Steering Committee. Each of Parties shall bear all expenses of its respective representatives and other participants in connection with Joint Steering Committee participation, including in connection with the attendance of any meetings thereof.

7.1.2 Functions The Joint Steering Committee shall perform the following functions: (a) coordinate the activities of the Parties and the Qualified Service Providers under the Development Plan; (b) recommend changes to the Development Plan, if applicable; (c) establish scientific and development teams for the execution of the Development Program as it sees fit, and settle any disputes or disagreements that are unresolved by any such teams; (d) serve as the governing body of all activities under this Agreement; and (e) perform such other functions as appropriate to further the purposes of the collaboration under this Agreement as determined by the Parties.

7.1.3 Meetings; Action by Written Consent. The Joint Steering Committee will meet as needed, but not less than [\*\*], and the members shall determine the form (e.g., in-person, telephone or video conference), timing, frequency and location of meetings. In principle, the meetings shall alternately take place in Amsterdam and Madrid. Representatives of either AMT or Digna or their Affiliates who are not members of the Joint Steering Committee may attend meetings of the Joint Steering Committee as agreed to by a representative member of the other Party. Any action required or permitted to be taken at any meeting of the Joint Steering Committee may be taken without a meeting if all members of the Joint Steering Committee consent thereto in writing, and such writing is filed with the minutes of proceedings of the Joint Steering Committee.

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7.1.4 Decision Making. Any approval, determination or other action agreed to by unanimous consent of the members of the Joint Steering Committee or their deputies present at the relevant Joint Steering Committee meeting shall be the approval, determination or other action of the Joint Steering Committee, *provided* at least one representative of each of Digna and of AMT are present at such meeting. The Joint Steering Committee will work in good faith to resolve any disputes that may arise among its members, *provided that* in the event any deadlock cannot be resolved in good faith by the Joint Steering Committee during a [\*\*] business day period following initial submission of any approval, determination or other action to the Joint Steering Committee, then the



issue shall be submitted to the Chief Executive Officers of AMT and Digna jointly who shall work in good faith to resolve any such dispute within [\*\*] business days. The joint decision of the Chief Executive Officers of AMT and Digna shall be deemed the approval, determination or action of the Joint Steering Committee. Any issue that cannot be resolved in good faith by the Chief Executive Officers of AMT and Digna within [\*\*] business days after the Joint Steering Committee has submitted the issue to them, shall be submitted to arbitration in accordance with Article 17 of this Agreement.

7.1.5 Minutes. The Joint Steering Committee shall keep accurate minutes of its deliberations which shall record all proposed decisions and all actions recommended or taken. The Secretary shall be responsible for the preparation of draft minutes. Draft minutes shall be sent to all members of the Joint Steering committee within [\*\*] business days after each meeting. All records of the Joint Steering Committee shall at all times be available to both Digna and AMT.

## ARTICLE 8 FUNDING OF DEVELOPMENT PROGRAM

8.1 Development Program. In consideration for the execution of the Development Program by Digna and its Qualified Service Providers (including the Hospital), AMT shall pay to Digna an aggregate amount of € 1,000,000 (one million euro) to be paid in installments as follows:

Upon signature of this Agreement	[**]
[**]	[**]
[**]	[**]
[**]	[**]
Total	€1,000,000

8.2 The funding set forth in Section 8.1 does not take into account the costs of Phase II/III Clinical Trials which are not within the scope of this Agreement and are subject to further agreement between the Parties (acknowledging that AMT may decide to have the Phase II/III Clinical Trials conduct by Third Parties). The funding set forth in Section 8.1, furthermore, does not take into account any preclinical work or Clinical Trials not described in the Development Plan that the Joint Steering Committee may decide to execute in order to facilitate the filing for Regulatory Approvals for the Product. In the event that the Joint Steering Committee decides to

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have such additional preclinical work or Clinical Trials conducted by Digna or its Qualified Service Providers, the Parties shall in good faith agree on the amount of the additional funding to be paid by AMT to Digna.

8.3 Payment to Digna. All amounts under this Article 8 shall be paid by AMT to Digna within [\*\*] days after receipt of the respective invoice. Digna shall be responsible for the apportionment of the sums paid to Digna hereunder to the Qualified Service Providers engaged by Digna in the execution of the Development Plan pursuant to the agreements executed between Digna and such Qualified Service Providers and for payment of the same according to that apportionment.

8.4 Taxes. All payments to Digna under the terms of the Agreement are expressed to be exclusive of value added tax howsoever arising and AMT shall, if required, pay to Digna in addition to those payments all value added tax for which Digna is liable to account in relation to any supply made or deemed to be made for value added tax purposed to this Agreement on receipt of a tax invoice or invoices from Digna. The Parties shall in such event closely cooperate to have such tax amounts refunded to AMT under applicable tax treaties.

Transfer of amounts. Payments made to Digna under this Agreement shall be made by wire transfer to the following account of Digna:

Bank: BBVA,  
Bank address: Avda. Carlos III, 33, 31004 Pamplona  
Swift Code: BBVAESMMXXX  
IBAN: ES89 0182 5000 8802 0156 0335

or any other bank account that may be notified by Digna to AMT from time to time.

8.5 EU Funding. The Parties acknowledge that they have applied for a so called European FP7 grant to cover (part of) the costs of the Clinical Trials. Digna acknowledges that the terms and conditions of the consortium agreement to be entered into between the partners to that FP7 project should not negatively affect the rights of AMT under this Agreement. Therefore, Digna and AMT shall negotiate and agree with all partners under said FP7 project clauses in the consortium agreement to the effect that Foreground (as defined in the FP7 grant agreement) generated by such partners shall be transferred to AMT. Any Foreground generated by Digna and [Hospital] under the FP7 project shall be considered Results as defined in this Agreement and AMT shall be the exclusive owner thereof.

## ARTICLE 9 SOPS / REPORTING / RESULTS

9.1 SOPs. Digna shall provide AMT with its own Standard Operation Procedures (SOPs) regarding the (organization) of preclinical studies and Clinical Trials within [\*\*] days after the Effective Date. Digna, furthermore, shall give AMT the right to review the SOPs of each Qualified Service Provider that Digna wishes to engage for the execution of its activities. AMT acknowledges that such SOPs are proprietary and confidential information of the respective Qualified Service Provider.

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9.2 Report / Data. Digna shall within [\*\*] months after completion of each activity under the Development Plan, provide the members of the Joint Steering Committee (or ensure that the Qualified Service Provider provides the members of the Joint Steering Committee) with a written report describing in sufficient detail the methodology used, the Results generated, an analysis of the Results and conclusions drawn from the Results.

9.3 Keeping data and records. Digna shall ensure that the Qualified Service Provider that execute the activities assigned to Digna under the Development Plan prepares and provides AMT with a database of accumulated data from all preclinical studies and/or Clinical Trials of the Product and of adverse information for the Product. AMT shall keep the trial master file with the essential documents relating to the Clinical Trial as well as any relevant records for at

least [\*\*] years after the completion of such activity / Clinical Trial in accordance with the most recent guidelines and regulations on clinical trials in the field of gene therapy (including the detailed EU guidelines on good clinical practice specific to advanced therapy medicinal products of December 3, 2009). The documents to be included in the trial master file shall, for each preclinical study and/or Clinical Trial, be provided by Digna to AMT within [\*\*] months after completion of the final report relating to such preclinical study and/or Clinical Trial.

9.4 Access. To the extent permitted by the Spanish Data Protection Act, Digna shall ensure that all Qualified Service Providers and Affiliates provide AMT access to all such data, to the extent necessary to meet or comply with any regulations or other requirements of the FDA, EMEA or other Regulatory Authorities, in each case with respect to Regulatory Approvals or other regulatory purposes

9.5 Ownership of Results. AMT shall exclusively own and have title, right and interest in and to the Results. AMT may, at its discretion, file patent applications for the Results in its name and at its expense.

## ARTICLE 10 CONFIDENTIALITY / PUBLICATION

10.1 General. Each Party undertakes to keep strictly confidential the Confidential Information and to use it only for the purpose of this Agreement.

10.2 No disclosure / restriction to use. Each Receiving Party undertakes to not disclose the Confidential Information to any third party except to those of its officers and employees who need to have access to the same. Each Party shall, before disclosing any Confidential Information to any of its officers or employees, make each such person aware of such restrictions and shall procure that such persons comply with such restrictions. If the Receiving Party wishes to disclose Confidential Information to any consultant or advisor who is not an officer or employee it shall obtain the Disclosing Party's prior written consent and shall furnish to the Disclosing Party with a confidentiality agreement signed by such consultant or advisor on the same terms and conditions of this Agreement.

10.3 Exceptions. The obligations to maintain confidentiality and to respect the restriction of the use shall not apply where, as properly evidenced by documentation: (a) is or becomes patented, published or otherwise becomes publicly known other than by acts of the Party obligated not to disclose such Confidential Information in contravention of this Agreement;

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(ii) can be shown by written documents to have been disclosed to the Receiving Party by a Third Party, *provided*, that such information was not obtained by such Third Party directly or indirectly from the other Party under this Agreement; (iii) prior to disclosure under this Agreement, was already in the possession of the Receiving Party; or (iv) can be shown by written documents to have been independently developed by the Receiving Party without use of the other Party's Confidential Information or breach of any of the provisions of this Agreement.

10.4 Permitted disclosure. Notwithstanding the above obligations of confidentiality and non-use a Recipient Party may:

10.4.1 disclose Confidential Information to a Regulatory Authority as reasonably necessary to obtain Regulatory Approval in a particular jurisdiction to the extent consistent with the licenses granted under terms of this Agreement; and

10.4.2 disclose Confidential Information: (a) to the extent such disclosure is reasonably necessary to comply with the order of a court; or (b) to the extent such disclosure is required to comply with a legal requirement, including to the extent such disclosure is required in publicly filed financial statements or other public statements under rules governing a stock exchange (e.g. the rules of the Netherlands, United States Securities and Exchange Commission, NASDAQ, NYSE, UKLA or any other stock exchange on which securities issued by either Party may be listed); provided, to the extent possible bearing in mind such legal requirements and subject to the next subsequent sentence of this Section 10.4, such Party shall provide the other Party with a copy of the proposed text of such statements or disclosure [\*\*] Business Days in advance of the date on which the disclosure is to be made to enable the other Party to review and provide comments, which shall be followed as long as they are reasonable, unless a shorter review time is agreed. If the compliance with a legal requirements requires filing of this Agreement, the filing Party shall to the extent possible seek confidential treatment of portions of this Agreement from the relevant competent authority and shall provide the other Party with a copy of the proposed filings at least [\*\*] Business Days prior to filing for the other Party to review any such proposed filing. Each Party agrees that it will obtain its own legal advice with regard to its compliance with legal requirements and will not rely on any statements made by the other Party relating to such legal requirements

10.4.3 disclose Confidential Information by filing or prosecuting Patent Rights, the filing or prosecution of which is contemplated by this Agreement, without violating the above secrecy provision; it being understood that publication of such filings occurs in some jurisdictions within [\*\*] months of filing, and that such publication shall not violate the above secrecy provision;

10.4.4 disclose Confidential Information to such Recipient Party's Affiliates, contractors (including clinical researchers) distributors, licensee's, agents, consultants, as such Recipient Party reasonably determines is necessary to receive the benefit of this Agreement or to fulfill its obligations pursuant to this Agreement; provided, however, any such persons must be obligated to substantially the same extent as set forth in Section 10.2 to hold in confidence and not make use of such Confidential Information for any purpose other than those permitted by this Agreement;

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10.4.5 disclose Confidential Information, only to the extent reasonably required, (i) to its actual or potential investment bankers; (ii) to existing and potential investors in connection with an offering or placement of securities for purposes of obtaining financing for its business and to actual and prospective lenders for the purpose of obtaining financing for its business; and (iii) to a bona fide potential acquirer or merger partner for the purposes of evaluating entering into a merger or acquisition, provided, however, any such persons must be obligated to substantially the same extent as set forth in Section 10.2 to hold in confidence and not make use of such Confidential Information for any purpose other than those permitted by this Agreement; and

10.4.6 Disclose Confidential Information to its legal advisers for the purpose of seeking advice.

## ARTICLE 11 PUBLICATION

11.1 Publication of Background IP and Confidential Information. Neither Digna nor AMT shall submit for written or oral scientific publication any manuscript, abstract or the like which includes Confidential Information or Background IP of the other Party (including joint IP) without first obtaining the prior written consent of the other Party, which consent shall not be unreasonably withheld and Digna shall ensure best reasonable efforts to procure that CIMA Parties

comply with the provisions of this Article 11. The contribution of each Party, if any, shall be mentioned in all publications or presentations by acknowledgement or co-authorship, whichever is appropriate.

11.2 **Publication of Results.** The Party wishing to submit any manuscript, abstract or the like regarding the Results for publication shall provide the other Party (the “**Reviewing Party**”) with a copy of any proposed publication which contains Results to the Reviewing Party at least [\*\*] days before the date of submission of the proposed publication to any publisher.

11.3 Within [\*\*] days of receipt of the proposed publication under Section 11.2, the Reviewing Party shall either:

(a) provide written consent to the proposed publication; or

(b) reasonably require to remove the Reviewing Party’s Confidential Information or Background IP whereby AMT shall always have the right to remove Confidential Information regarding its production and manufacturing processes;

(c) reasonably require that the proposed publication be delayed or amended (without altering the scientific meaning of the publication) to enable a patent application to be filed regarding any Results contained in the proposed publication. The delay or amendment required by the Reviewing Party shall be reasonable and in any event, any delay required shall be no longer than three months from the date the other Party provided a copy of the proposed publication to the Reviewing Party.; and/or

(d) provide comments and/or amendments in relation to the proposed publication which will be reasonably considered and incorporated by the other party into the publication.

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11.4 In the event of any dispute regarding the proposed publication, the Parties shall resolve such differences in good faith through the Joint Steering Committee as provided for in Article 7.

11.5 If within [\*\*] days of receipt of the proposed publication the Reviewing Party does not:

11.5.1 provide consent under Section 11.3(a); or

11.5.2 request a delay or amendment under Section 11.3(c),

the Reviewing Party shall be deemed to have given consent for the proposed publication for the purposes of Section 11.5(a), always provided that no Party is entitled to publish Confidential Information or Background IP of the other Party, even in absence of a reasonable request as referred to in Section 11.3(b).

11.6 Digna ensures that the CIMA Parties shall comply with this Article 11 as if they were a Party thereto.

11.7 Digna shall use its best efforts to include in the agreements with the Qualified Service Providers clauses regarding publications rights that substantially resemble the ones set forth in this Article 11, including a reasonable review time by Digna (and consequently, AMT) of any proposed scientific publication on the Clinical Trial by such Qualified Service Provider.

## **ARTICLE 12 REPRESENTATIONS AND WARRANTIES / INDEMNIFICATION / INSURANCE**

12.1 **Mutual Warranties.** Each Party represents and warrants to the other Parties that:

(a) It has full power to extend the granted hereunder and perform its obligations hereunder;

(b) It has full power and authority to enter into this Agreement and has taken all necessary action on its part required to authorize the execution and delivery of this Agreement;

(c) The execution, delivery and performance of this Agreement and its compliance with the terms and provisions hereof does not conflict with, or result in a breach of any of the terms and provisions of, or constitute a default under any agreement to which any Party is a party; and

(d) The execution, delivery and performance of this Agreement by each Party does not require the consent, approval or authorization of or notice, filing or registration with Regulatory Authority.

12.2 **Indemnification by AMT.** AMT indemnifies and holds harmless Digna, its Qualified Service Providers (including the Hospital and the Principal Investigator) and their employees against all claims and proceedings (to include any settlements or ex gratia payments

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made with the consent of the Parties hereto and reasonable legal and expert costs and expenses) made or brought (whether successfully or otherwise) by or on behalf of Clinical Trial subjects and (or their dependants) against Digna or a Qualified Service Provider or their employees for personal injury (including death) to Clinical Trial subjects arising out of or relating to the administration of the Product under investigation or any clinical intervention or procedure provided for or required by the Protocol to which the Clinical Trial subjects would not have been exposed but for their participation in the Clinical Trial. The above indemnity by AMT shall not apply to any such claim or proceeding (i) to the extent that such personal injury (including death) is caused by the negligent or willful misconduct of Digna or a Qualified Service Provider, their employees or agents and/or (ii) to the extent that such personal injury (including death) is caused by the failure of a Qualified Service Provider or its employees to conduct the Clinical Trial in accordance with the Protocol and/or GCP.

12.3 **Indemnification by Digna.** Digna, as Sponsor, indemnifies and holds harmless AMT and its employees against all claims and proceedings (to include any settlements or ex gratia payments made with the consent of the Parties hereto and reasonable legal and expert costs and expenses) made or brought (whether successfully or otherwise) (i) to the extent that such personal injury (including death) is caused by the gross negligent or willful misconduct of Digna, a

Qualified Service Provider or their employees and/or (ii) to the extent that such personal injury (including death) is caused by the failure of Digna or a Qualified Service Provider to conduct the Clinical Trial in accordance with the Protocol and/or GCP.

Digna's liability arising out of or in connection with any breach of this contract or any act or omission of Digna or its Qualified Service Providers in connection with the Clinical Trial shall, save for willful misconduct or gross negligence of Digna or its Qualified Service Providers, in no event exceed the amount of fees paid by AMT according to clause 8.

12.4 The indemnified Party hereunder shall as soon as reasonably practicable following receipt of notice of such claim or proceeding, notify the indemnifying Party hereunder in writing of a claim and shall, upon the indemnifying Party's request permit the indemnifying Party to have full care and control of the claim or proceeding using legal representation of its own choosing.

12.5 Insurance. Each of AMT and Digna will take out appropriate insurance cover their potential liability under this Article 12 (including, Digna in its capacity of Sponsor of the Clinical Trials, an clinical trial insurance as required by the Spanish Regulatory Authorities). Each Party shall produce to the Qualified Service Provider and to each other on request, copies of insurance policies or other evidence thereof together with evidence that such policies remain in full force and effect. The terms of any insurance or the amount of cover shall not relieve AMT or Digna of any liabilities under this Agreement.

## ARTICLE 13 TERM / TERMINATION

13.1 Term. This Agreement shall commence on the Effective Date and shall continue until completion of the Development Plan, save for premature termination in accordance with this Article 13.

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13.2 Termination. Notwithstanding any other provision hereof, each Party may forthwith terminate this Agreement:

(a) as a result of a material breach or default in the performance of any obligation, condition or covenant of this Agreement by the other Party or Parties, if such default or noncompliance shall not have been remedied within [\*\*] days after receipt by the defaulting Party of a notice thereof from the other Party (whereby it is agreed that failure to comply with the Protocol or GCP by Hospital and/or the Principal Investigator is deemed to be a material breach by Digna); or

(b) if the other Party is declared insolvent or, whether voluntarily or involuntarily, is declared bankrupt, or if such Party becomes permanently unable to perform its obligations hereunder for reasons other than suspension of payment or bankruptcy, such as, for example, liquidation, dissolution or winding-up.

13.3 Termination by AMT for other reasons. AMT may, furthermore, terminate this Agreement upon two (2) months written notice in the event that the Joint Steering Committee (subject to Section 7.1.4.) decides to cease further development of the Product for safety, efficacy or technical feasibility or commercial reasons.

13.4 Effect of Termination.

13.4.1 Effects of termination by AMT pursuant to Section 13.2. In the event of termination by AMT pursuant to Section 13.2 the following applies (notwithstanding AMT's rights by law or equity):

(a) AMT remains sole owner of the Results.

(b) The Development Plan will be terminated as per the earliest possible date and the funding obligations of AMT as set forth in Article 8 will terminate. AMT will be free to further develop and Commercialize by itself or with Third Parties at its discretion and is released from any further payments to Digna under this Agreement.

13.4.2 Effects of termination by Digna pursuant to Section 13.2 or by AMT pursuant to Section 13.3. In the event of termination by Digna pursuant to Section 13.2 or by AMT pursuant to Section 13.3, the following applies (notwithstanding Digna's rights by law or equity):

(a) AMT shall assign to Digna co-ownership of the Results. Digna shall be exclusively entitled to use and Commercialize the Results on a royalty-free basis for the further development and Commercialization of a product for the treatment or prevention of the Disorder. This right is with the right to sublicense. Each of AMT and Digna may, on a royalty-free and non-exclusive basis, without the right to sublicense, use the Results for the treatment or prevention of disorders other than the Disorder. User rights of the Joint Patent Rights in the event of termination of this Agreement are set forth in the License Agreement.

(b) AMT shall cease development, manufacture and Commercialization of Products as treatment or prevention of the Disorder;

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(c) The Development Plan will be terminated as per the earliest possible date. The funding obligations of AMT as set forth in Article 8 will terminate as per the effective date of termination of this Agreement.

(d) AMT will, if so requested by Digna, enter into good faith discussions with Digna regarding the terms and conditions under which AMT, or a third party engaged by AMT, would be willing to manufacture the Product for Digna or its sublicensee.

13.5 Surviving Articles. Articles 10, 11, 12, 16 and 17 shall survive expiration or termination of this Agreement for any reason.

## ARTICLE 14 ASSIGNMENT

Save as otherwise provided in this Agreement no Party shall without the prior written consent of the other assign the benefit and/or burden of this Agreement provided always that AMT may assign this Agreement to an Affiliate of AMT or any purchaser of the whole or part of AMT's assets or to a company with which

AMT is merging, provided that such Affiliate, purchaser or merger company undertakes to Digna to be bound by the terms of this Agreement.

## ARTICLE 15 NOTICES

Any notices required or provided under this Agreement shall be in writing and shall be given by facsimile or by certified mail addressed to the applicable Party as set out below:

### AMT

Mr. Piers Morgan  
PO Box 22506  
1100DA Amsterdam  
The Netherlands

### Digna

Mr. Pablo Ortiz Betés  
DIGNA BIOTECH S.L.AVDA. PÍO XII, 22, OFICINA 2.31008 PAMPLONA,  
NAVARRA  
Spain

## ARTICLE 16 GOVERNING LAW

The validity construction and performance of this Agreement shall be governed by the laws of The Netherlands.

## ARTICLE 17 JURISDICTION

All disputes arising out of or in connection with this Agreement shall be finally settled under the Rules of Arbitration of the International Chamber of Commerce by one or more arbitrators

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appointed in accordance with said Rules. The arbitration to take place in Paris and to be conducted in English.

## ARTICLE 18 MISCELLANEOUS

### 18.1 Interpretation.

18.1.1 If an ambiguity or a question of intent or interpretation arises with respect to this Agreement, this Agreement shall be construed as if drafted jointly by the Parties and no presumption or burden of proof shall arise favoring or disfavoring any Party by virtue of the authorship of any provisions of this Agreement.

18.1.2 Whenever the context may require, any pronoun shall include the corresponding masculine, feminine and neuter forms. The words “include”, “includes” and “including” shall be deemed to be followed by the phrase “without limitation.” The word “will” shall be construed to have the same meaning and effect as the word “shall.” Unless the context requires otherwise, (A) any definition of or reference to any agreement, instrument or other document herein shall be construed as referring to such agreement, instrument or other document as from time to time amended, supplemented or otherwise modified (subject to any restrictions on such amendments, supplements or modifications set forth herein or therein), (B) any reference to any laws herein shall be construed as referring to such laws as from time to time enacted, repealed or amended, (C) any reference herein to any Person shall be construed to include the Person’s successors and assigns, (D) the words “herein”, “hereof and “hereunder”, and words of similar import, shall be construed to refer to this Agreement in its entirety and not to any particular provision hereof, (E) any reference herein to the words “mutually agree” or “mutual written agreement” shall not impose any obligation on either Party to agree to any terms relating thereto or to engage in discussions relating to such terms except as such Party may determine in such Party’s sole discretion and; (F) all references herein to Articles, Sections or Schedules shall be construed to refer to Articles, Sections and Schedules of this Agreement.

18.2 Force Majeure. Neither Party shall be held liable or responsible to the other Party nor be deemed to have defaulted under or breached this Agreement for failure or delay in fulfilling or performing any term of this Agreement when such failure or delay is caused by or results from causes beyond the reasonable control of the affected Party, including fire, floods, embargoes, war, acts of war (whether war is declared or not), insurrections, riots, civil commotions, strikes, lockouts or other labor disturbances, acts of God or acts, omissions or delays in acting by any governmental authority or the other Party; *provided, however*, that the Party so affected shall use Commercially Reasonable Efforts to avoid or remove such causes of non-performance, and shall continue performance hereunder with reasonable dispatch wherever such causes are removed. Each Party shall provide the other Parties with prompt written notice of any delay or failure to perform that occurs by reason of force majeure. The Parties shall mutually seek a resolution of the delay or the failure to perform in good faith.

18.3 Severability. Each Party hereby agrees that it does not intend to violate any public policy, statutory or common laws, rules, regulations, treaty or decision of any government agency or executive body thereof of any country or community or association of countries. Should one or more provisions of this Agreement be or become invalid, the Parties hereto shall

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substitute, by mutual consent, valid provisions for such invalid provisions which valid provisions in their economic effect are sufficiently similar to the invalid provisions that it can be reasonably assumed that the Parties would have entered into this Agreement with such valid provisions. In case such valid provisions cannot be agreed upon, the invalidity of one or several provisions of this Agreement shall not affect the validity of this Agreement as a whole, unless the invalid provisions are of such essential importance to this Agreement that it is to be reasonably assumed that the Parties would not have entered into this Agreement without the invalid provisions.

### 18.4 Exhibits. The Exhibits to this Agreement form an integral part of this Agreement.

18.5 Entire Agreement. This Agreement (including the Exhibits thereto) contains the entire understanding of the Parties with respect to the subject matter hereof and supersedes all prior or contemporaneous oral or written agreements of the Parties with respect to the subject matter hereof, including the Previous Agreements. This Agreement may be amended, or any term hereof modified, only by a written instrument duly executed by both Parties hereto.

18.6 Headings. The captions to the several Articles and Sections hereof are not a part of this Agreement, but are merely guides or labels to assist in locating and reading the several Articles and Sections hereof.

18.7 Independent Contractors. It is expressly agreed that the Parties to this Agreement shall be independent contractors and that the relationship between the Parties shall not constitute a partnership, joint venture or agency. Neither Party shall have the authority to make any statements, representations or commitments of any kind, or to take any action, which shall be binding on the other(s), without the prior consent of the other Parties to do so.

18.8 Waiver. Except as expressly provided herein, the waiver by either Party hereto of any right hereunder or of any failure to perform or any breach by the other Party shall not be deemed a waiver of any other right hereunder or of any other failure to perform or breach by said other Party, whether of a similar nature or otherwise, nor shall any singular or partial exercise of such right preclude any further exercise thereof or the exercise of any other such right.

18.9 Counterparts. This Agreement may be executed in one more counterparts, each of which shall be deemed an original, but all of which together shall constitute one and the same instrument.

18.10 Benefit. Nothing in this Agreement or the agreements referred to herein, expressed or implied, shall confer on any person other than the Parties hereto or thereto, or their respective permitted successors or assigns, any rights remedies, obligations or liabilities under or by reason of this Agreement, the agreements referred to herein, or the transactions contemplated herein or therein.

18.11 Further Assurances. Each Party shall, as and when requested by another Party, do all acts and execute all documents as may be reasonably necessary to give effect to the provisions of this Agreement.

[Remainder of page intentionally left blank]

IN WITNESS WHEREOF, the Parties have executed this Collaborative Development Agreement as of the date first written above.

/s/ Pablo Ortiz Betés  
Digna Biotech S.L.  
By: Pablo Ortiz Betés  
Title: Director General

/s/ Piers Morgan  
Amsterdam Molecular Therapeutics (AMT) B.V.  
By: Piers Morgan  
Title: Chief Financial Officer

**EXHIBIT 1**  
**DEVELOPMENT PLAN**

**Project Plan:**

The aim of this collaboration is to develop an AIP product and to deliver a data package for the product that is suitable for the submission and approval by the European and North American regulatory authorities. The product should be approved for the general treatment of AIP (both males and females.) The quality of the project has to comply with the national and internationally accepted quality standards. A quality plan, defining the CRO selection, quality inspections, auditing etc will be agreed between the parties. It is acknowledged by the Parties that the timelines set out below were drafted at the beginning of the negotiation process on this Agreement and the License Agreement and that, in view of the fact that several months passed since then, the Joint Steering Committee will update the timelines as soon as possible after the signature of this Agreement.

Task No	Task	Timelines	Delivering party
1	[**]	[**]	[**]
2	[**]	[**]	[**]
3	[**]	[**]	[**]
4	[**]	[**]	[**]
5	[**]	[**]	[**]
6	[**]	[**]	[**]
7	[**]	[**]	[**]
8	[**]	[**]	[**]
9	[**]	[**]	[**]
10	[**]	[**]	[**]

## Conditions and Specifications

### PoC in pre-clinical models

- PoC in rodent disease model
- PoC in non-human primates, based on agreed protocol
- Go-no-go
  - efficacy in rodent disease model
  - 10% hepatic transduction and/or PBDG levels [\*\*] fold above normal in rodents
  - 10% hepatic transduction in non-human primates

### GLP Toxicology

- Scientific advice from a regulatory body (AEMPS and/or EMA) for safety and toxicology package (duration, number of species, biodistribution, inclusion of immunosuppressants, risk of integration, germ line transmission)
- GLP toxicology study in rodents rats or mice (transduction will be compared between mice and rats, only if transduction levels are equal in rats to the transduction levels in mice, rats will be used), 180 days according to relevant EU guideline (EMA/CHMP/GTWP/125459/2006) with interim sacrifices for acute toxicity [\*\*], and delayed toxicity at early time point [\*\*]. The need for an additional late time point [\*\*] will be discussed. Biodistribution studies (including gonadal tissues) should provide data on all organs, whether target or not, needs Scientific advice.
- Toxicology data in a non-rodent species, using the route of administration intended in the clinic duration similar to rodent study, including bio-distribution (including gonadal tissues) and efficacy endpoints. This work is not included in the amount payable by AMT to DIGNA under section 8.1. Costs hereof will be agreed by the Joint Steering Committee and will be funded by AMT. Needs Scientific advice. Details to be agreed by the Joint Steering Committee.
- GLP germline transmission in female and maternal transmission study in mice, according to the relevant European guidelines, will be conducted in case DNA is found in gonadal tissue. Details to be agreed by the Joint Steering Committee. (AMT may decide to conduct additional germline transmission in female and maternal transmission study in mice even if these are not strictly required for the Phase I/II trial, in such case, AMT, at its own choice, would have the option to fund such a study and DIGNA will ensure that such a study is done as agreed).
- GLP germ line transmission study in male rabbits (duration exceeding [\*\*] cycles of spermatogenesis, [\*\*]) will be conducted at additional cost. (The quotation is required prior the final contract signature).

Toxicology study design will take into account:

- Identification of potential target organs of biological activity (for efficacy studies- i.e. liver) and of potential target organs of toxicity,
- Eventual concomitant medication (e.g. immunosuppressants, standard co-medication),
- Environmental risk/shedding, has to be included in the tox studies. The Joint Steering Committee will evaluate and agree whether this would be required for the for Phase I/II trial

- Analysis of appropriateness of surrogate markers of efficacy/safety,
- Any other relevant issues as agreed by the Joint Steering Committee.

Deliverable: Toxicology study report suitable for the submission the regulatory authority.

### Observational, pre-intervention study/studies

- DIGNA and AMT agree on the need of good quality patient's data (clinical and biochemical) before entering the interventional study in order to adequately assess the efficacy of the product. For this reason, patient's to be included in the Phase I/II clinical trial will be followed for a period between [\*\*] months before entering into the trial. The type of data to be collected, clinical and analytical, will be agreed by the Joint Steering Committee.
- Little is known of the natural history of this disease. The medical history, phenotypic presentation, natural evolution of AIP patients has to be explored and documented (for the 'group of AIP patients') over an adequate time period. In addition, a baseline against which to measure intra-individual post-administration efficacy and safety has to be established per individual study subject. Main investigator of University Clinic will conduct a observational preparatory study to provide baseline information on the course of the disease by recording episodes AIP, abdominal pain, hospitalizations, extent of any possible known or unknown to be related to AIP symptomatology, incidence of (adverse) clinical events per year, etc., sufficient to provide a clinical picture to obtain a baseline data and to determine how efficacy will be show during the trial. Also, to allow for inclusion of [\*\*] eligible and willing subjects into the subsequent interventional trial, a sufficient number of subjects needs to included in the observational preparatory study (not everybody would be suitable or willing to progress). The Joint Steering Committee will evaluate and agree how it can best ensure that sufficient number of subjects is included.
- The period of observation of the first observational trial should be at an estimated minimum of [\*\*] months on an individual basis, and at least [\*\*] months for the group/cohort (of to-be treated subjects) average. In parallel to the intervention study, subjects entered into the observational study but not enrolled in the intervention study should continue in the observational study up to [\*\*] months.
- Observational study needs to comply with the nationally and internationally accepted standard, to make sure that the data are suitable for the regulatory submission for marketing authorisation. This will be done if so requested by AMT at an additional cost of [\*\*] euros. The following conditions should be met:
  - The study should conform to all industry standards, to guidance of Institutional Review Board/Independent Ethics Committee ("IRB/IEC") approval(s), to all relevant guidance relating to medicines and clinical trials from time to time in force including, but not limited to, the ICH Harmonized Tripartite Guidelines for Good Clinical Practices (E6).
  - AMT to be provided with a full list of clinical SOPs and copies of the applicable SOPs as requested by AMT prior to the conduct of the study.
  - Both parties to agree on the content of the study audit plan prior to the start of the study, including any relevant quality plan.
  - The Principle Investigator (PI) will attempt to secure timely consent from all patients, to be rolled over into an AMT owned by AIP Registry, in which among others natural evolution and long term safety and efficacy after gene therapy will be captured, at a point to be determined.
  - Go/no go for subsequent intervention study/studies: sufficient disease presentation, manifestation & complication data and sufficient information on natural disease evolution is collected and sufficient individual patient baseline data is acquired, to allow the conclusion that any data to be collected in the interventional study can be used to judge interventional product safety and gauge clinical efficacy using suitable, determinable endpoints. The go/no go criteria will also include:

- Site and protocol feasibility that supports the conduct and execution of the proposed study according the agreed timeline

- Regulatory and ethical approval and
- Actual progress that is consistent with achieving agreed accrual targets and timelines

#### Phase I/II

- GCP & guidelines compliant as per above
- The clinical phase I/II should include an estimated minimum [\*\*] patients that are administered the gene therapy drug, and are followed up and clinically assessed for at least [\*\*] months following drug administration.
- The formal study follow up will be at least [\*\*] years post administration per individual subject; interim analysis and reporting will be planned for, at a minimum once immediately after all subjects have had their [\*\*] months study visit.
- The PI has to commit to a [\*\*] year follow up of those [\*\*] treated patients, at minimum for safety and efficacy assessments each year during the first [\*\*] years and [\*\*] thereafter, and needs to commit upfront to maintaining adequate study and patient data documentation, and to keep any source documents secure, for [\*\*] years after study drug administration.
- The clinical trial must include all biochemical, imaging, clinical and functional assays, as well as any other relevant additional assays, to assess the disease state and change therein over time, as well as the phenotypic disease variation, as well as the overall clinical and psychosocial or other health status or change therein over time of the individual trial subjects, both before, during and following drug administration.
- Study monitoring and source data verification will be carried out and documented to industry accepted standards.
- AMT will have, at the latest [\*\*] months after the obtaining of each individual data point, access to and exclusive use of the data for the purposes of ultimately obtaining marketing authorizations, in Europe and/or any other country or region in the world. This data access is needed early on to engage in discussion with relevant regulatory bodies representatives.
- Within [\*\*] months after the completion of each individual study or study phase, AMT will receive a copy of the essential documents on request and copies of the study database. Upon completion, AMT will receive a copy of the final, audited, clinical trial database, with data, and those aspects of the Trial Master File that are necessary to file for MAA.
- Study data only published with approval and agreement of the Liaison Committee

#### **EXHIBIT 2**

PROTOCOL (to be attached once final)

Confidential Materials omitted and filed separately with the Securities and Exchange Commission. A total of 137 pages were omitted. [\*\*]



Confidential Materials omitted and filed separately with the  
Securities and Exchange Commission. Double asterisks denote omissions.

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INSTITUT PASTEUR

(1)

and

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AMSTERDAM MOLECULAR THERAPEUTICS (AMT) BV

(2)

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DEVELOPMENT and MANUFACTURING  
AGREEMENT

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THIS DEVELOPMENT AND MANUFACTURING AGREEMENT (this “Agreement”) is effective as of January 7<sup>th</sup>, 2011 (“Effective Date”).

BY AND BETWEEN

ON THE ONE HAND

- (1) INSTITUT PASTEUR a non profit private foundation organized under the laws of France with offices at 25-28 rue du Docteur Roux, 75 724 Paris Cedex 15, France, VAT FR 65 775 684 897 (“Institut Pasteur”), acting herein in the name and behalf of the Consortium (“Consortium” and each designated individually as “Consortium Member”) which has been organized under an agreement by and between the following members:

L’INSTITUT NATIONAL DE LA SANTE ET DE LA RECHERCHE MEDICALE, *Etablissement Public à caractère Scientifique et Technologique*, organized under the laws of France, having its principal offices at 101 rue de Tolbiac, 75013 Paris, (“INSERM”),

INSERM TRANSFERT, Société Anonyme, organized under the laws of France, registered under RCS Paris n° 434 033 619 having its principal offices at 7, rue Watt - 75013 Paris, (“INSERM-TRANSFERT”),

L’ECOLE NATIONALE VETERINAIRE ET DE L’AGROALIMENTAIRE ET DE L’ALIMENTATION DE NANTES ATLANTIQUE, centre d’expérimentation sur l’animal en thérapie génique et cellulaire, organized under the laws of France, having its principal offices at Atlanpole - La Chantrerie - 44 307 NANTES, (“ONIRIS”),

INSTITUT PASTEUR a non profit private foundation organized under the laws of France with offices at 25-28 rue du Docteur Roux, 75 724 Paris Cedex 15, France, VAT FR 65 775 684 897 (“INSTITUT PASTEUR”),

L’ASSOCIATION FRANCHISE CONTRE LES MYOPATHIES, l’Association Française contre les Myopathies, an *association* governed by the law of July 1, 1907, *reconnue d’utilité publique de droit privé*, organized and existing under the laws of France, having its principal office at l’Institut de Myologie, 47-83 boulevard de l’Hôpital, 75651 Paris Cedex 13, (AFM)

(“the Consortium represented herein by “Institut Pasteur”)]

And

ON THE OTHER HAND

- (2) AMSTERDAM MOLECULAR THERAPEUTICS (AMT) BV a company incorporated under the laws of the Netherlands, with offices at P.O.Box 22506 - 1100 DA Amsterdam, The Netherlands, (“AMT”).

(each, a “Party.” and together the “Parties”)

BACKGROUND:

- (A) Sanfilippo Syndrome IIIB (“Sanfilippo B”) is a lysosomal storage disorder caused by a deficiency of the enzyme a-N-acetylglucosaminidase (NaGlu), resulting in a severe degenerative pathology of central nervous system.
- (B) Institut Pasteur, acting herein in the name and behalf of the Consortium which has been organized to execute a program of research and development relating to Sanfilippo B, according to an agreement by and between INSTITUT PASTEUR, INSERM, INSERM-TRANSFERT, ONIRIS and AFM. In this context, Institut Pasteur intends to carry out Phase I/II and Phase II/III clinical trials for the gene therapy treatment of Sanfilippo B with Product (as defined below) that has been manufactured by AMT in accordance with AMT’s proprietary manufacturing technology.

- (C) This Agreement sets out the terms and conditions on which AMT will develop the manufacturing process for the Product and supply one or two clinical Batches to Institut Pasteur.

IT IS NOW AGREED AS FOLLOWS:

1 Definitions

- 1.1 The following capitalized terms, whether used in the singular or plural, shall have the meanings assigned to them below for purposes of this Agreement:
- 1.2 “Acquisition Cost” means the actual price paid by AMT to any Third Party (net of any discounts, rebates, credits or the like) for any Raw Materials, Consumables, Wearables used in the manufacture of the Product under this Agreement, plus [\*\*] percent ([\*\*]%) of such actual price to cover AMT’s acquisition and storage costs for such materials together with all shipping and handling costs and customs duties incurred and paid by AMT to that Third Party in connection with the acquisition of such materials.
- 1.3 “Additional Services” means any service that is not contained in this Agreement and that requires a Change Order according to Section 3.6(c) in order to authorize AMT to commence the same or any service specifically identified as an Additional Service in this Agreement, for which the Parties will determine in good faith the amount to be paid for the performance of such Additional Services according to the prices specified in Exhibit A.
- 1.4 “Affiliate” means, with respect to either Party, any other corporation or business entity that directly, or indirectly through one or more intermediaries, controls, is controlled by or is under common control with such Party. For purposes of this definition, the term “control” means direct or indirect ownership of more than fifty percent (50%) of the securities or other ownership interests representing the equity voting stock or general partnership or membership interest of such entity or the power to direct or cause the direction of the management or policies of such entity, whether through the ownership of voting securities, by contract, or otherwise.

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- 1.5 “AMT Confidential Information” means the MPR, the Batch Disposition Documentation, any Manufacturing Documentation, the Manufacturing Information, the AMT Background Information and all elements of the Manufacturing Process provided from time to time by AMT to Institut Pasteur together with all technical and other information, whether patented or unpatented, relating to the AMT Facility and/or AMT processes, methods, operations, technologies, forecasts and business information and all other data and information that is disclosed or supplied to, or used on behalf of, Institut Pasteur by AMT pursuant to this Agreement, or of which Institut Pasteur may become aware through the presence of its employees or agents at AMT offices or at the AMT Facility, including, without limitation, trade secrets, know-how, processes, concepts, experimental methods and results and business and scientific plans and information and facility layout. All portions of documents and records describing or relating to AMT Intellectual Property shall be deemed to be AMT Confidential Information.
- 1.6 “AMT Facility” means the facilities operated by AMT at *Meibergdreef 45 and 61, 1105 BA Amsterdam, The Netherlands*.
- 1.7 “AMT Background Information” means all technical know-how and information known to AMT and all AMT Intellectual Property (including all AMT Confidential Information) (a) which is incorporated into the Manufacturing Process, or (b) that is necessary to the practice of the Manufacturing Process and/or for the utilisation of the Deliverables.
- 1.8 “Batch” means a specific quantity of Product or other material produced from a single Run.
- 1.9 “Batch Disposition Documentation” means the following documentation associated with the production and testing of a given Clinical Batch: batch production records, Release Statements, the Certificate of Analysis and the Certificate of Compliance. Such documentation shall be deemed to be AMT Confidential Information disclosed to Institut Pasteur pursuant to Section 17, except under the provisions of Sections 11.8 and 14.3.
- 1.10 “Certificate of Analysis” means a document prepared by AMT listing in relation to each Batch the tests performed by AMT or approved Subcontractors, the specifications and test outcomes.
- 1.11 “Certificate of Compliance” means a document prepared by AMT: (i) listing the manufacturing date, unique Batch number, and quantity of Product in such Batch, (ii) certifying that such Batch was manufactured in accordance with the Master Production Record and cGMP and (iii) certifying that all Investigative and Corrective Action Reports are completed and approved. The Parties shall from time to time agree upon a format or formats for the Certificate of Compliance to be used under this Agreement.

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- 1.12 “cGMP” means the regulatory requirements for current good manufacturing practices in EC Directives 2003/94/EC and 2005/28/EC, as applicable to the responsibilities specified in the Quality Agreement, the agreed upon Project Plan or this Agreement, as well as any applicable ICH (International conference on harmonization) guidelines, as well as any additional regulatory agency requirements needed to seek registration in the EU, such as Part II of Volume IV of the EU Guide to Good Manufacturing Practice, as any of the foregoing may be amended from time to time and anything which replaces or supersedes the same from time to time.
- 1.13 “Change Order” means a document mutually approved in writing by both Parties in accordance with the procedures set forth in Section 3.6 that describes in detail an amendment or modification to the Project and/or the Project Plan.
- 1.14 “Clinical Batch” means a Batch product from a Clinical Run.
- 1.15 “Clinical Run” means a Run manufactured in accordance with the Master Production Record and cGMP and used to create Product for use in human clinical trials.

- 1.16 “Confidential Information” means Institut Pasteur Confidential Information and/or AMT Confidential Information, as the context requires.
- 1.17 “Conforming Product” means Product that conforms to all of the warranties set forth in Section 15.2(c).
- 1.18 “Consumable” means all bags, liners, filters, membranes and other single use or regularly replaced materials that are required to perform the Manufacturing Process (excluding Raw Materials and Wearables) in accordance with this Agreement.
- 1.19 “Deliverables” means all or any of the deliverables set out in Exhibit A and “Deliverable” has a cognate meaning.
- 1.20 “Delivery Dates” means the dates under which the Deliverables have to be transmit to Institut Pasteur according to the Exhibit A.
- 1.21 “Development Batch” means a Batch produced from a Development Run.
- 1.22 “Development Run” means a Run performed in accordance with the Master Production Record and any approved validation protocol(s) to confirm and/or to document the operability and reproducibility of the Manufacturing Process at the AMT Facility.
- 1.23 “EMA” means the European Medicines Agency or any successor agency thereto or replacement thereof, whether in whole or in part.
- 1.24 “Governmental Authority” means any national, multi-national, regional, state or local regulatory agency, department, bureau, or other governmental entity.

- 1.25 “Institut Pasteur Background Information” means all technical know-how and information known to Institut Pasteur and all Institut Pasteur Intellectual Property (including all Institut Pasteur Confidential Information) and in particular any and all scientific, technical or test data including research data, clinical pharmacology data, chemistry, manufacturing and control data (analytical and quality control data, stability data), preclinical data and clinical data relating to the Product.
- 1.26 “Institut Pasteur Change Request” means a AMT document used to accomplish amendments and modifications to documents which are part of AMT’s cGMP document system, including but not limited to MPRs, standard operating procedures and Materials Specifications.
- 1.27 “Institut Pasteur Confidential Information” means any clinical data and information, business plans, regulatory and Product strategies and all technical and other information, whether patented or unpatented, relating to Institut Pasteur processes, test methods, operations, technologies, formulations, forecasts and business information, Institut Pasteur Background Information and all other data and information that is disclosed or supplied to AMT by or on behalf of Institut Pasteur pursuant to this Agreement, and/or that is produced by AMT for the Institut Pasteur in the performance of this Agreement.
- 1.28 “Intellectual Property” means all Patents, copyrights, rights in and to databases, rights in and to trade secrets and know-how and all other intellectual property rights that are owned or controlled by a Party, including all applications and registrations with respect thereto, and all rights to apply for the same, in each case subsisting at any time anywhere in the world. For purposes of this Section 1.30, “controlled by” means possession of the any and all rights to grant a license or sublicense.
- 1.29 “Investigative and Corrective Action Reports” or “ICAR” means the document that is used to record the investigation of, as well as the review and disposition of, a failure related to a cGMP manufacturing process or system.
- 1.30 “Manufacturing Documentation” means all documents and records describing or otherwise related to the Manufacturing Process, other than those embodied in the Master Production Record.
- 1.31 “Manufacturing Process” means the production process for the manufacture of Product developed by AMT pursuant to this Agreement.
- 1.32 “Master Production Record” or “MPR” means the document, proposed by AMT and subsequently approved in writing by Institut Pasteur. The AMT proposed document, or revisions thereto, will be approved by signature and dating by at least one representative of AMT’s quality unit and will be prepared in accordance with Section 6.41 of Volume 4 of the “EU Guidelines to Good Manufacturing Practice, Medicinal Products for Human and Veterinary Use, Part II, Basic Requirements for Active Substances used as Starting Materials”, and which specifies:

- (a) The name of the Product being manufactured and an identifying document reference code; and
- (b) A complete list of raw materials and intermediates designated by names or codes sufficiently specific to identify any special quality characteristics; and
- (c) A complete set of raw material specifications listing specific methods used for confirming compliance with specification; and
- (d) A complete list of resin, filtration membranes, filter cartridges etc., designated by names or codes sufficiently specific to identify manufacturer, type and/or model, as appropriate; and
- (e) An accurate statement of the quantity or ratio of each raw material or intermediate to be used, including the unit of measure. Where the quantity is not fixed, the calculation for each batch size or rate of production should be included. Variations to quantities will be included where justified; and
- (f) The production location(s) and production equipment to be used, including the;

- (i) The unique identification number of the production location(s) (for example - suite/room numbers, laminar flow cabinets etc.); and
- (ii) The unique identification number of each production piece of equipment to be used; and
- (iii) Confirmation of the production equipment's approved calibration status; and
- (g) Detailed production instructions, including the:
  - (i) Sequences to be followed; and
  - (ii) Initial process set-point and acceptable operating ranges of all processing parameters to be used; and
  - (iii) Sampling instructions for in-process control samples, in-process control methods with their acceptance criteria where appropriate; and
  - (iv) Time limits for completion of individual processing steps, hold times and/or the total process time limit, where appropriate; and
- (h) Detailed finished Product Release Specification testing instructions, including the:

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- (i) Sampling instructions and Release Testing Specifications indicating the release acceptance criteria; and
- (ii) Release Testing methods; and
- (i) Where appropriate, special notations and precautions to be followed, or cross references to these; and
- (j) Instructions for the storage of the Product and isolated intermediate products (for example - Inclusion Bodies) to assure their suitability for use, including the labelling and packaging materials and special storage conditions with time limits and hold times, where appropriate.
- 1.33 "Material Safety Data Sheet" means a data sheet which contains information on the chemical, physical, and toxicological properties of a potentially hazardous product and recommendations for proper handling, storage, disposal, and emergency response.
- 1.34 "Materials Specification" or "MS" means a document detailing the specifications for each Raw Material or Consumable, each as mutually approved by the Parties in writing.
- 1.35 "NDA" means a new drug application for the Product, or any equivalent filing thereto, including, without limitation, a biologics license application filed with the FDA, a marketing authorization application filed with the EMA, or any equivalent application filed with Health Canada, and any supplements or amendments to any of the foregoing. The NDA shall also include equivalent filings in such other jurisdictions as the parties mutually agree upon in writing pursuant to a Change Order.
- 1.36 "Non-Conforming Product" means Product that fails to conform to all of the warranties set forth in Section 15.2(c) and "Non-Conformity" shall have a cognate meaning.
- 1.37 "Patents" shall mean, with respect to an invention, any patent or patent application, and any patent issuing therefrom, together with any extensions, reissues, reexaminations, substitutions, renewals, divisions, continuations and continuations-in-part thereof, and any patent or patent application claiming priority from any application in common with any such patent containing a disclosure substantially similar to that of any such patent, all to the extent the foregoing contain claims covering such invention.
- 1.38 "Pre-Process Development" shall mean tasks associated with defining ranges, conditions and criteria to be used in Process Development.
- 1.39 "Process Development" shall mean the demonstration, through Development Runs, that the Manufacturing Process is operable as defined.

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- 1.40 "Product" means the AAV5 vector-based gene a-N-acetylglucosaminidase (NaGlu) therapy for the treatment of Sanfilippo B disease
- 1.41 "Project" means the full range of manufacturing and other services to be provided by AMT under this Agreement.
- 1.42 "Project Plan" means the scope of work for development and manufacturing and overall project scope, together with the allocated costs payable by Institut Pasteur attached as Exhibit A hereto and hereby incorporated into this Agreement, as may be updated from time to time by the Parties mutually agreeing in writing to adopt a revised version.
- 1.43 "Quality Agreement" means the quality criteria to be agreed between the Parties which, when executed by the Parties, shall be incorporated into this Agreement as an Exhibit C. The Quality Agreement shall not be an Additional Service.
- 1.44 "Quality Review and Approval" means AMT's review and approval in accordance with cGMP, by AMT's quality assurance department, of a Batch and the associated Batch Disposition Documentation.
- 1.45 "Raw Material" means all ingredients, solvents and other components of the Product in the amounts required to perform the Manufacturing Process (excluding any Consumables and Wearables) in accordance with this Agreement.

- 1.46 “Reference Materials” means Product that is generated from a Run that is well characterized, packaged and stored in a controlled manner, and used as a standard or reference for analytical testing purposes.
- 1.47 “Regulatory Authority” means any or all of the FDA, the EMA and/or Health Canada and any body or bodies which may replace or supersede the same from time to time whether in whole or in part. “Regulatory Authority” shall also include equivalent bodies in such other jurisdictions as the parties mutually agree upon in writing pursuant to a Change Order.
- 1.48 “Regulatory Filing” means any or all applications, submitted to Regulatory Authorities for the purpose of registering the Product or the Manufacturing Process as required by statute, and any amendments or supplements thereto, and any other filings required by the Regulatory Authorities relating to the manufacture, testing, sale or distribution of the Product, including, without limitation, an NDA.
- 1.49 “Release Specification” means in respect of the Product, the document to be agreed by the Parties listing tests to be performed by AMT or approved Subcontractors and the acceptance criteria for these tests such criteria to be based on the “Tentative Acceptance Criteria” set out in Exhibit A.
- 1.50 “Release Statements” means a document prepared by AMT that provides confirmation that Product has met its assigned Release Specification(s).

- 1.51 “Results” means know-how, ideas, results, concepts, materials, works, inventions and discoveries that are made, conceived, reduced to practice or developed in the course of performing under or resulting from this Agreement by AMT or its employees, Sub-contractors or agents, but expressly excluding the Institut Pasteur Background Information, the Institut Pasteur Intellectual Property, the AMT Background Information and the AMT Intellectual Property.
- 1.52 “Run” means a single complete operation of all, or a discrete portion of, the Manufacturing Process at the AMT Facility.
- 1.53 “Shipping Guidelines” means AMT’s written procedures, as approved by Institut Pasteur in writing, that describe the methods of packaging, preserving, monitoring and shipping, any and all Institut Pasteur property, including the Product.
- 1.54 “Storage Guidelines” means AMT’s procedures, as approved by Institut Pasteur in writing, that describe the methods of packaging, preserving, monitoring and storing any and all Institut Pasteur property, including the Product.
- 1.55 “Subcontractor” means any Third Party that AMT contracts with to perform any services or meet any obligations that are required under the terms and conditions of this Agreement.
- 1.56 “Third Party” means any person other than Institut Pasteur (and consequently any person other than the membres of the Consortium : INSERM, INSERM-TRANSFERT, ONIRIS, AFM) or AMT. Genethon is a Third Party.
- 1.57 “Waste” shall mean any waste material, pollutant and/or contaminant of any kind including, without limitation, any routine process waste or any by-product arising from any activities conducted pursuant to this Agreement.
- 1.58 “Wearables” means any non-sterile coverings or protective gear used by AMT employees or agents in the course of the performing the development and manufacturing services hereunder, including without limitation gloves, coveralls, booties, eye shields and the like.
- 1.59 “Working Day” means any day on which clearing banks are open for business in the Netherlands and France provided that any reference to a “day” shall be a calendar day.
- 1.60 Each of the following definitions are found in the body of this Agreement, or elsewhere, as indicated below:

Defined Term	Section
Agreement	Preamble
Institut Pasteur	Preamble
AMT	Preamble
Delivery Sample	15.3(a)
Development	2.1

Disputed Matter	21.1
Effective Date	Preamble
Executive Oversight Committee or EOC	3.2
Expert Determination	13.2
Force Majeure Event	20.1
Manufacturing Information	11.8
Notified Party	17.6.1
Notifying Party	17.6.1
Parties	Preamble
Party	Preamble
Patent Claim	14.5
Project Manager	3.2
Referral Notice	21.2
Registration	11.1
Replacement Product	13.3
Term	19.1

- 2.1 Process Development and Assay Development. Subject to the terms and conditions set forth in this Agreement, during the Term, Institut Pasteur hereby retains AMT to, and AMT shall use commercially reasonable efforts, to perform the Process Development and Assay Development (together, the “Development”) of the Manufacturing Process in accordance with this Agreement and Exhibit A, at the AMT Facility, and Institut Pasteur shall pay AMT for such Development work, all in accordance with this Agreement as set out in the Project Plan. Institut Pasteur shall pay AMT for such Development services in accordance with Exhibit A and Article 8.
- 2.2 AMT shall carry out the Development as defined in the Project Plan with reasonable skill and care and no less than the level of skill and care to be reasonably expected of a provider of such services, and in compliance with all applicable laws and regulations including cGMP (if applicable). For the avoidance of doubt, it shall not be considered a breach of this Agreement by AMT if any objective of the Project Plan is not achieved:
- (a) so long as AMT uses commercially reasonable efforts to perform its obligations; or
  - (b) in relation to all stages of the Project Plan, due to delay caused or contributed to by Institut Pasteur.
- 2.3 The Parties acknowledge that, having regard to the fact that the work to be performed hereunder is by its nature developmental, AMT does not guarantee to Institut Pasteur the achievement of a successful outcome for the objectives under

the Project Plan, but will use commercially reasonable efforts to carry out such stages of the Project Plan and to ensure timely success.

- 2.4 Manufacture. Subject to the terms and conditions, AMT shall use commercially reasonable efforts to meet the agreed estimated timelines and Delivery Dates set forth in the Project Plan and shall manufacture the Clinical Batches within the scope of the Project Plan with reasonable skill and care and no less than the level of skill and care to be reasonably expected of a provider of such services, and in compliance with all applicable laws and regulations including cGMP (if applicable). Institut Pasteur shall purchase such Clinical Batches from AMT in accordance with Exhibit A and Article 8.
- 2.5 All right, title and interest on any and all works performed under this Agreement, and in particular the Clinical Batches and Results, to the exclusion of the Manufacturing Process, shall be owned by the Institut Pasteur upon payment for such works under Article 8.
- 2.6 The steps of the Project Plan which shall be subject to go/no go decisions from Institut Pasteur, and in particular after the completion of each Batch are described in Section 3 and Exhibit A.

3 Project Plan; Project Management

- 3.1 Project Plan. In order to enable the Parties to fulfill their respective obligations under this Agreement, AMT shall implement and perform its obligations as set out in the Project Plan. The Project Plan may be amended by agreement of the Parties in accordance with Section 3.6. Adherence to the Delivery Dates set out in the Project Plan is contingent in part on each Party’s reasonably expedient turnaround of document reviews and approvals where such review and approval is necessary.
- 3.2 Project Management; Appointments. The day-to-day interactions and management with respect to the Project will be performed by two project managers, one appointed by each Party and each one having the authority to manage the Project in conjunction with the other project manager and to further the aims of the Parties day-to-day (each, a “Project Manager”). As part of their duties, the Project Managers shall be responsible for monitoring and revising the Project Plan (in accordance with the procedures set forth in Section 3.6), establishing operating guidelines for the Project, defining communication formats, forming and approving Project teams and monitoring the general progress of the Project. The Project Managers shall be appointed by each respective Party no later than [\*\*] days following the Effective Date. AMT shall not remove or replace its Project Manager, except where such person has left the employment, where such Person has taken a leave of absence, where such Person is out on disability or sick leave for more than a [\*\*]-week period, or if Institut Pasteur agrees in writing to such removal or replacement, without giving not less than [\*\*] days notice in writing to AMT and subject to same skills.

- 3.3 Follow up meeting. The Project Managers shall meet regularly to discuss any issues, problems or other matters, and as much as necessary but at least [\*\*] times per year.
- 3.4 The Parties shall appoint a committee in charge in particular of any Go/No Go decisions. The committee shall be composed of members of both Parties, and shall meet regularly, at least [\*\*] a year, and shall draft minutes of its meeting. This committee shall take the Go/No Go decisions of any [major steps] of the services performed under this Agreement. The decisions shall be taken by the committee, provided however that the final decision shall be taken by [\*\*]. The committee may assign an expert in order to answer any questions relating to the services performed under this Agreement.
- 3.5 The Project Managers will prepare minutes of the meeting within [\*\*] Working Days of the meeting and distribute these minutes to the Parties for review and approval. Either AMT or Institut Pasteur shall be deemed to approve such minutes if it does not object to them within [\*\*] Working Days of submission of the relevant minutes. In case of any problems, the management of each Party shall be consulted.
- 3.6 Project Changes.
- (a) Project Plan Changes. If at any time either Party is of the reasonable opinion that the Project Plan requires updating (otherwise than as a consequence of any breach of this Agreement by either Party), then such Party shall notify immediately in writing the other Party. The Parties shall discuss and agree in good faith as soon as practicable, but in any event within [\*\*] Working Days after receipt of such

notice a revised Project Plan signed on behalf of each of the Parties. Upon execution of the new Project Plan described above, the new timelines set forth therein shall govern.

- (b) AMT Initiated Changes. Before AMT may amend the Project Plan, AMT shall prepare a Change Order describing in detail the nature of such change(s), and propose such Change Order to Institut Pasteur for Institut Pasteur's review and written approval. All approved Change Orders shall be approved by each Party only by signature by the Project Manager of each Party or by such other authorized representatives of AMT and Institut Pasteur that the Project Managers may designate in writing to the other Party. If any changes contemplated by a Change Order will have a financial or other impact on the Project, AMT shall provide Institut Pasteur with a written description of such impact in the Change Order. If Institut Pasteur approves the Change Order, Institut Pasteur shall pay AMT any additional charges as detailed in the Change Order. Any such charge increase shall be priced on a milestone or time and materials basis, as mutually agreed upon by the Parties. Upon AMT and Institut Pasteur's approval of the Change Order, this Agreement shall be deemed amended in accordance with such Change Order.

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- (c) Institut Pasteur Initiated Changes. Institut Pasteur shall have the right to request relevant modifications to the Project and/or the Project Plan by providing notice thereof to AMT. Upon receipt of such notice, AMT shall generate a Change Order in accordance with the process described in Section 3.5(b), and submit such Change Order to Institut Pasteur for Institut Pasteur's review and approval. If Institut Pasteur approves the Change Order in accordance with Section 3.5(b), Institut Pasteur shall pay AMT any additional charges as detailed in the Change Order. Upon Institut Pasteur's approval of such Change Order, this Agreement shall be deemed amended in accordance with such Change Order.

#### 4 Manufacturing and Processing Activities

- 4.1 Quality Agreement. The Quality Agreement shall be agreed and executed within [\*\*] months following the Effective Date and shall specify certain testing, storage, release, cGMP, regulatory and other quality assurance requirements relating to manufacture and shipment of Product by AMT under this Agreement. AMT shall comply with the Quality Agreement at all times in carrying out its obligations under this Agreement.
- 4.2 AMT Facility. All Products to be manufactured for Institut Pasteur hereunder shall be manufactured solely by AMT at the AMT Facility or by an AMT Affiliate if such AMT Affiliate is approved in writing by Institut Pasteur. Any Affiliate proposed by AMT must be capable of manufacturing Product in accordance with cGMP, the Master Production Record, the Release Specification and the Quality Agreement. AMT shall be responsible for all scheduling related to the manufacturing at AMT Facility or at AMT Affiliate facilities. Without prejudice to the foregoing, AMT's right to contract an Affiliate or a sub-contractor to manufacture Product is subject to AMT remaining, at all times, solely liable to Institut Pasteur for all of the Affiliate or sub-contractor's activities and for any failure by such an Affiliate to comply with the relevant terms of this Agreement.
- 4.3 Raw Materials and Consumables.
- (a) Procurement. AMT shall be responsible for the procurement of all commercially available Raw Materials, Wearables and Consumables necessary for the manufacture of the Product. AMT shall not be responsible for delays in the purchase and/or delivery of Raw Materials, Wearables and Consumables that occur despite AMT's implementation of the foregoing procedures and despite AMT using commercially reasonable efforts to avoid such delays. The cost of all Raw Materials, Wearables and Consumables that relate to activities included in the Project Plan are included in the sums payable by Institut Pasteur set out in the Project Plan.
- (b) Compliance with Release Specifications. All Raw Materials, Wearables and Consumables used in the Manufacturing Process shall comply with the applicable Materials Specifications, or as otherwise agreed in writing by the

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Parties. AMT or a Subcontractor approved in accordance with Section 4.5 shall perform testing and evaluation of the Raw Materials, Wearables and Consumables as required to meet the foregoing obligations.

- 4.4 Storage and Use of Materials and Product. All Raw Materials, Wearables and Consumables that are in AMT's control and are to be used in the manufacture of Product, as well as all product (other than Waste) from the Manufacturing Process and Product in AMT's control, shall be stored in accordance with the terms and conditions of the Storage Guidelines, the Material Specifications and/or the Master Production Record, or as otherwise mutually agreed in writing by AMT and Institut Pasteur.
- 4.5 Approval of Subcontracting. AMT shall not subcontract, sublicense or otherwise delegate all or any portion of its obligations under this Agreement without Institut Pasteur's prior written approval. Institut Pasteur may as a condition of giving such consent require that it is directly involved, to a reasonable degree, in monitoring the performance of any Subcontractor appointed by AMT. Notwithstanding the foregoing, AMT may subcontract certain non-essential or routine tasks without Institut Pasteur's consent, provided that they are tasks which would normally be sub-contracted by AMT in the normal course of its business and are performed in compliance with Good Industry Practice and, where applicable, cGMP (e.g., cleaning of cGMP suites, and maintenance and service of AMT Facility systems (e.g., Clestra, HEPA certification and electrical upgrades)), on a confidential basis.
- 4.6 Document Changes. Any requests by AMT or Institut Pasteur for changes to cGMP documentation, including the Master Production Record and any standard operating procedures, will be handled by the procedure in Section 3.6.

#### 5 Development Runs.

- 5.1 AMT shall perform Development Runs and manufacture Development Batches in accordance with the Project Plan, Delivery Dates and the Master Production Record. AMT shall provide Institut Pasteur with any Development Batches requested by Institut Pasteur that result from any partial or completed Development Runs. Institut Pasteur shall have the right to make whatever further use of such Development Batches as it shall determine, provided that such use does not violate any applicable laws, rules or regulations. In the event that AMT becomes aware that it

may not be able to achieve any Delivery Dates in respect of Development or the manufacture of Product it shall immediately notify Institut Pasteur and the reasons why, and the Parties will agree in good faith a new Delivery Date under reasonable time limits with regard to the Project Plan.

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6 Clinical Batches

- 6.1 An initial Clinical Batch will be manufactured in accordance with the Project Plan for the purposes for a Phase I/II study (“the First Clinical Batch”) in the quantity described in Exhibit A.
- 6.2 If the program progresses to a further Phase II/III study, then as provided in the Project Plan, a second Clinical Batch will be required (“the Second Clinical Batch”), in the quantity to be determined by the Parties.
- 6.3 Institut Pasteur shall notify AMT in writing that it requires a Second Clinical Batch at least [\*\*] months prior to the proposed Delivery Date of such Second Clinical Batch. If AMT does not agree to manufacture the Second Clinical Batch, AMT may terminate the Agreement in accordance with Sections 19.2(a) and the licence granted to Institut Pasteur in accordance with Clauses 11.8 to 11.11 shall take effect. If Institut Pasteur does not request the Second Clinical Batch Institut Pasteur may terminate the Agreement according to the procedure of Section 19.2 (d), but in which case no Manufacturing Information shall be provided to Institut Pasteur under Section 11.8 and no licence shall be granted to Institut Pasteur under Section 14.3 .

7 Deliveries

- 7.1 Delivery Date. For each Clinical Batch, the Delivery Date shall be the date established by the Parties. If AMT reasonably expects any delay in the Delivery Date agreed for either Batch, it shall promptly inform Institut Pasteur of such expected delay and shall use its commercially reasonable efforts to minimize the delay. Each Batch will be shipped in accordance with the Shipping Guidelines.
- 7.2 Delivery Terms. Within [\*\*] Working Days following Institut Pasteur’s acceptance of a Batch pursuant to Section 13.1, AMT shall make such Batch available to the designated carrier at AMT’s Facility in accordance with the Shipping Guidelines. Institut Pasteur shall arrange for shipment of each Batch within [\*\*] months after written notice of such availability is provided to Institut Pasteur. AMT shall provide storage for such Batch in accordance with the Storage Guidelines [\*\*] during such [\*\*] month period; provided, that any additional storage beyond such [\*\*] month period [\*\*] to Institut Pasteur as Additional Services . The risk of loss for each Batch shall be borne by Institut Pasteur from the date of shipment. Institut Pasteur shall be responsible for all appropriate approvals and consents of any Governmental Authority necessary for the transportation or shipment of such Batch.
- 7.3 No Storage of Product. Notwithstanding anything to the contrary contained in this Agreement, in no event shall AMT be required to store a Batch of Product for more than [\*\*] following the later of (i) Institut Pasteur’s acceptance thereof in accordance with this Agreement; and (ii) the grant of the last of any required

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approval(s) and/or consent(s) of any Governmental Authority necessary for the transportation or shipment of such Batch.

8 Payments

- 8.1 Payment for Development services. Institut Pasteur shall pay AMT for all Development services provided hereunder in accordance with Exhibit A.
- 8.2 Product Pricing. Institut Pasteur shall pay AMT for all manufacturing services used to produce Product for Institut Pasteur in accordance with Exhibit A.
- 8.3 Payment Terms. All invoices for work performed shall, in the case of work carried out pursuant to the Project Plan, be issued when the relevant milestone set out in the Project Plan has been completed and, in the case of Clinical Batches, be issued on the Delivery Date. All amounts due thereunder shall be due and payable within [\*\*] days after receipt of such invoice which shall be sent from AMT to Institut Pasteur after completion of the relevant milestones or delivery of the Clinical Batches. Payments shall be made by wire transfer or check in Euros. Institut Pasteur shall pay interest to AMT on any sums not paid to AMT on the date on which payment should have been made pursuant to the applicable provisions of this Agreement (“Due Date”) over the period from the Due Date until the date of actual payment (both before and after judgment) at the rate of [\*\*] per cent, above the base rate from time to time of Deutsche Bank plc (or, if less, the maximum rate allowed to be charged under applicable laws). Interest accrues and is payable from day to day.

9 Documentation

- 9.1 Regulatory Documentation. Within [\*\*] days following completion of the Quality Review and Approval for each Clinical Batch, but in any event not later than the Delivery Date, AMT shall provide Institut Pasteur with a copy of all applicable Batch Disposition Documents, which documents shall be in AMT’s standard formats in the form at Exhibit B unless otherwise mutually agreed to by the Parties, provided that such Batch Disposition Documents will not be considered as an Additional Service. Any Institut Pasteur requests for documents or other work product (other than the MPR, the Manufacturing Documentation and copies of Batch Disposition Documentation and any other documents or work product expressly required to be delivered under this Agreement) that do not exist as of the date of such request or other substantive requests for assistance in compiling any Regulatory Filing shall constitute Additional Services, and AMT shall notify Institut Pasteur of the same, and, if Institut Pasteur authorizes such services, AMT shall prepare a Change Order and invoice Institut Pasteur for such Additional Services.
- 9.2 Retention and Reserve Samples. AMT shall identify and retain certain reserve samples of all intermediate production samples generated in the production of a Clinical Batch as applicable, as set forth in the applicable Materials Specifications,



the applicable standard operating procedures, the Master Production Record, Section 15.3 or as otherwise agreed in writing by AMT and Institut Pasteur. After completion of the applicable Run, AMT shall inform Institut Pasteur of the availability of these samples. Institut Pasteur shall request these samples on conditions to be determined in good faith between AMT and Institut Pasteur. AMT shall retain and preserve, at its sole cost and expense, samples and standards of Product in accordance with the requirements of cGMP.

- 9.3 Analytical Testing. AMT, or a designated Subcontractor approved in accordance with Section 4.5, shall perform the analytical testing on Batches as set forth in the Master Production Record, or as otherwise agreed in writing by AMT and Institut Pasteur.
- 9.4 Accurate Documentation. Each Party shall use its reasonable efforts to provide complete and accurate records and documentation to the other Party in connection with any Deliverable and, to the extent applicable, in accordance with the Quality Agreement, or as otherwise agreed in writing by AMT and Institut Pasteur.

## 10 Manufacturing audits

- 10.1 Institut Pasteur shall have the right to perform, directly or through its representatives, certain manufacturing audits of the AMT Facility or the facilities used by any AMT Affiliate or sub-contractor to manufacture the Product as set forth in the Quality Agreement, or as otherwise agreed in writing by AMT and Institut Pasteur. All AMT personnel time and resources necessary to complete [\*\*] shall be provided at no cost to Institut Pasteur; however, any AMT personnel time and resources necessary to complete any additional manufacturing audits [\*\*] in that same [\*\*] years shall be invoiced to Institut Pasteur as Additional Services in accordance with the Project Rates. Institut Pasteur shall be responsible for all third party costs of all manufacturing audits, unless the audit identifies a breach by AMT of its obligations under this Agreement of such significance in which case, the costs of the audit shall be paid by AMT.

## 11 Regulatory matters

- 11.1 Permits. AMT shall secure and maintain, at its sole cost and expense, current governmental registrations, permits and licenses as are required from time to time by Governmental Authorities in order for AMT to perform all of its obligations under this Agreement (each, a “Registration”), for so long and insofar as is necessary to permit AMT to perform any of its obligations under this Agreement. AMT shall make copies of such Registrations and all related documents available for viewing by Institut Pasteur and its designees for inspection, upon reasonable request from Institut Pasteur. All copies will remain in AMT’s control.
- 11.2 Compliance with cGMPs; Monitoring of Records. As further described in the Quality Agreement, or as otherwise agreed in writing by AMT and Institut Pasteur, AMT shall monitor and maintain reasonable records respecting its compliance with

cGMPs, including the process of establishing and implementing the operating procedures and the training of personnel as are reasonably necessary to assure such compliance.

- 11.3 Records. AMT shall maintain all records required to be maintained by the terms and conditions of the Quality Agreement and by applicable law and regulation, including cGMP.
- 11.4 Regulatory Communications and Correspondence. Any and all communications from and to Regulatory Authorities related to the manufacture of the Product at the AMT Facility shall be handled in accordance with the terms and conditions of the Quality Agreement, or as otherwise agreed in writing by AMT and Institut Pasteur.
- 11.5 Regulatory Filings and Maintenance. Institut Pasteur shall prepare and maintain all Regulatory Filings.
- 11.6 Subject to Sections 11.7 to 11.11, because the Manufacturing Process is confidential to AMT, apart from the Batch Disposition Documentation, AMT shall not be obliged to provide any information relating to the Manufacturing Process to Institut Pasteur or any Third Party.
- 11.7 Whilst AMT is continuing to manufacture Product for Institut Pasteur under this Agreement, AMT shall, upon Institut Pasteur’s request, file information relating to the Manufacturing Process confidentially and directly with Regulatory Authorities or allow it to be cross-referenced in a confidential manner by the Regulatory Authorities for the purposes of supporting any Regulatory Filings made by Institut Pasteur relating to the Product.
- 11.8 If, pursuant to Section 6.3 and 19.2(a), AMT does not agree to manufacture the Second Clinical Batch then it shall, subject to Sections 11.9 to 11.11 and Section 14.3, provide to Institut Pasteur all the necessary information describing the Manufacturing Process and Manufacturing Documentation to allow a Third Party contracted by Institut Pasteur to manufacture the Product, as well as the Batch Disposition Documentation (the “Manufacturing Information”).
- 11.9 The appointment of such Third Party manufacturer shall require the consent of AMT, such consent not to be unreasonably withheld, in particular in relation to the treatment of the patients with the Product. Without limiting the foregoing, it shall be reasonable for AMT to refuse consent if the proposed Third Party manufacturer is Généthon or any of its Affiliates.
- 11.10 The Manufacturing Information is AMT’s Confidential Information and Institut Pasteur’s obligations in Section 17 shall apply to such Manufacturing Information without limit in time. Institut Pasteur shall impose on the Third Party manufacturer of the Product equivalent obligations to those set out in Section 17. Without limiting the foregoing, Institut Pasteur and any Third Party contracted by Institut Pasteur to manufacture the Product shall not disclose any Manufacturing Information to Généthon or any of its Affiliates.

- 11.11 The Manufacturing Information or any part of it can only be used in relation to the manufacture of Product and for no other purpose whatsoever.
- 11.12 For the avoidance of doubt, any additional activities which are not included in the Project Plan which are specific to the Institut Pasteur's Regulatory Filing are subject to a Change Order as described in Section 3.6(c).
- 11.13 Ownership of Regulatory Filings. Institut Pasteur shall be the sole owner of all Regulatory Filings (except those filed by AMT in accordance with Section 11.7) and all governmental approvals obtained by Institut Pasteur from any Regulatory Authority with respect to the Product.
- 11.14 Health and Safety Information. Each Party shall promptly notify the other of any information or notice of which it becomes aware concerning the safety or efficacy claims of the Product, including, without limitation, any threatened or pending action by any Regulatory Authority. Institut Pasteur shall be responsible for handling all complaints and communications from Regulatory Authorities with respect to the Product. AMT shall cooperate in resolving such complaints and responding to such communications to the extent they pertain to the Product and such cooperation is reasonably requested by Institut Pasteur. Institut Pasteur shall reimburse AMT for all reasonable costs and expenses incurred by AMT in connection with the performance of AMT's obligations under this Section 11.14 except to the extent that any such complaint or communication arises from any breach of this Agreement by AMT or through the grossly negligent or willful act or omission of AMT, its employees, sub contractor or agents.
- 11.15 Accident Reports. Each Party shall report to the other promptly all material accidents related to the manufacture, handling, use or storage of any Raw Materials or Product, which could delay the performance of this Agreement.

12 Quality Assurance; Quality Control

- 12.1 Responsibility for Quality Assurance and Quality Control. Responsibility for quality assurance and quality control of Product shall be allocated as set forth in the Quality Agreement.
- 12.2 Qualification a Validation of AMT Facility; Utilities. AMT shall maintain cGMP qualification and validation, as appropriate, of the AMT Facility, as well as the utilities and equipment used in the manufacture of Product at the AMT Facility, and shall make relevant reports applicable thereto (redacted to remove information not related to the manufacture of Product) available to Institut Pasteur for review at AMT's Facility, at Institut Pasteur's reasonable request.
- 12.3 AMT shall be responsible to manufacture the Clinical Batch and the Development Batch, according to the quality requirements decided by both Parties. AMT shall be the sole responsible for the release of any batches in accordance with the terms of Exhibit A "QP Release", following the regulations in force.

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13 Non-conformance

- 13.1 Notice of Nonconformity. AMT shall provide Institut Pasteur's quality assurance department with copies of completed Batch Disposition Documentation, and shall endeavor to do so within [\*\*] Working Days of Quality Review and Approval. Within [\*\*] Working Days after Institut Pasteur's receipt of such Batch Disposition Documentation, Institut Pasteur shall determine by review of such Batch Disposition Documentation whether or not, the given Clinical Batch conforms to the warranties set forth in Section 15.2(c) and (d), provided that AMT provides timely answers to information requests and resolution of issues arising from Institut Pasteur's review of such Batch Disposition Documentation. If within the [\*\*] Working Days period, Institut Pasteur's quality assurance department makes a determination that Institut Pasteur believes such Batch to be nonconforming, Institut Pasteur shall have the right to reject such Batch in its entirety and shall notify AMT in writing within such [\*\*] Working Days period. Such written notice shall specify the manner in which the Clinical Batch fails to conform to the warranties set forth in Section 15.2(c) and (d). If Institut Pasteur does not submit written notice of rejection within such [\*\*] Working Days period, such Batch will, subject to the remaining provisions of this Section 13.1, be deemed a Conforming Product and accepted by Institut Pasteur. In the event that Institut Pasteur desires to accept such Batch prior to the end of the [\*\*] Working Days period, Institut Pasteur will fax written notice of such acceptance to AMT's Project Manager.
- 13.2 Within [\*\*] days of receipt of a notice from Institut Pasteur pursuant to Section 13.1, AMT shall notify Institut Pasteur whether or not it accepts or disputes Institut Pasteur's assertions that the Batch is non-conforming. In the event of an unresolved dispute the Parties shall appoint an independent testing laboratory or other expert ("Expert"), reasonably acceptable to both Parties, and subject to confidentiality provisions comparable to those set out in Section 17 below, to undertake the relevant analysis, which analysis shall be performed in compliance with the applicable laws of the relevant governmental authority, to determine whether the Batch conformed or did not conform to the warranty in Section 15.2(c) and (d). Both Parties agree to cooperate with the Expert's reasonable requests for assistance in connection with its analysis hereunder. The test results obtained from such Expert shall be conclusive and binding upon the Parties, absent manifest error.
- 13.3 No AMT Liability. If it is determined by agreement of the Parties (or in the absence of such agreement, by the Expert in accordance with Section 13.2 that either (i) the Batch is Conforming Product, or (ii) there is a nonconformity with respect to such Batch but the nonconformity was not caused by AMT's breach of the warranties set forth in Section 15.2(c) and (d), then AMT shall have no liability to Institut Pasteur with respect to such Batch, and Institut Pasteur shall pay for such Batch and for the fees associated with the Expert and the Batch shall be treated in all other respects under this Agreement as in conformance with all of the warranties set forth in Section 15.2(c) and (d) of this Agreement.

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- 13.4 AMT Liability for Non-Conforming Product; Replacement; Termination. If following the receipt of a notice from Institut Pasteur pursuant to Section 13.1, it is determined by agreement of the Parties (or in the absence of such agreement, by an Expert appointed in accordance with Section 13.2) that such Batch is Non-Conforming Product and such non-conformance was caused by AMT's negligence or willful misconduct, or which arises out of or results from any breach of this Agreement by AMT, then (A) (i) AMT shall, promptly, replace such Non-Conforming Product with Conforming Product (the "Replacement Product") or (ii) if AMT is unable to provide Institut Pasteur with Replacement Product within [\*\*] days after such determination, Institut Pasteur shall be entitled to terminate this agreement on notice in accordance with

Section 19.2(d); and (B) if an Expert was retained, AMT shall be responsible for the fees and expenses of the Expert, and (C) AMT shall replace free of charge (or, where appropriate, reimburse Institut Pasteur for the cost of) all Raw Materials and Consumables utilized in the production of the Non-Conforming Product. Delivery of Replacement Product shall be at no additional cost to Institut Pasteur assuming Institut Pasteur previously paid the purchase price for the Non-Conforming Product, in which case no additional payment for the Replacement Product will be required. If in accordance with the procedures in Sections 13.1 and the preceding terms of this Section 13.4, the Replacement Product is determined by agreement of the Parties (or in the absence of such agreement, by an Expert appointed in accordance with Section 13.2) that the Replacement Product is Non-Conforming Product and such non-conformance was caused by AMT's negligence or willful misconduct, or which arises out of or results from any breach of this Agreement by AMT then Institut Pasteur shall be entitled to terminate this agreement on notice in accordance with Section 19.2(d).

- 13.5 Cooperation in Investigations; Disposition of Non-Conforming Product. Each Party shall act in good faith and shall cooperate with the other Party and with an Expert appointed pursuant to Section 13.2 or 13.3. AMT shall dispose of any Non-Conforming Product in accordance with Section 4.4 and all relevant laws, rules and regulations with respect to such disposal, at AMT's cost if AMT was liable for the nonconformity in accordance with Section 13.3

#### 14 Ownership and License Grants

- 14.1 Licenses to AMT. During this Agreement, Institut Pasteur hereby grants to AMT a royalty-free, non-exclusive, non-transferable, non-sub-licensable, license under any and all Institut Pasteur Intellectual Property and Institut Pasteur Background Information that is necessary for AMT to perform its obligations under this Agreement, including, without limitation, the manufacture of Product for Institut Pasteur but for the sole purposes of implementing the Project under this Agreement.
- 14.2 No License to Institut Pasteur. Apart from the right to use the Product delivered to Institut Pasteur under this Agreement, the right to cross reference the documentation filed by AMT with the Regulatory Authorities in accordance with

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Section 11.7 and the right to use the Manufacturing Information in accordance with Sections 11.8 to 11.11 and 14.3, Institut Pasteur has no right or licence to any AMT Background Information or other Intellectual Property of AMT.

- 14.3 If pursuant to a request of Institut Pasteur according to Sections 6.3, AMT does not agree to manufacture the Second Clinical Batch and consents to the appointment of the Third Party manufacturer for the Product under Section 11.8, then AMT grants Institut Pasteur (and consequently AMT grants to INSERM, INSERM-TRANSFERT, ONIRIS and AFM as members of the Consortium represented herein by Institut Pasteur, expressly acknowledged by the Parties that GENETHON is not a member of the Consortium) a non-exclusive, royalty-free licence to its AMT Background Information on the terms of Sections 11.8 to 11.11 solely to the extent necessary to allow Institut Pasteur and/or a Third Party manufacturer contracted by Institut Pasteur to manufacture Product for Institut Pasteur for the treatment of Sanfilippo B. AMT may terminate this licence if Institut Pasteur or its Third Party manufacturer breaches the terms of this licence or Sections 11.8 to 11.11 inclusive. Under the provisions of this Section 14.3, it shall be the responsibility of Institut Pasteur to obtain any Third Party licences that it needs to manufacture Product for the Second Clinical Batch, if any.
- 14.4 Trademarks. Notwithstanding anything to the contrary contained in this Agreement, neither Party shall acquire any license to use nor any ownership rights in the trade marks, trading names, trading styles, brands or logos of the other.
- 14.5 Third Party Intellectual Property. If after the Effective Date any Third Party files and serves on AMT, or threatens AMT or any Consortium Member to file, pursue or maintain any claim, lawsuit, charge, complaint or other action alleging infringement by AMT of a Third Party Patent based on the manufacture, testing, use, import, offer for sale or sale of the Product (each a "Patent Claim") then:
- (a) AMT so notified by the Third Party shall notify Institut Pasteur;
  - (b) AMT shall immediately cease working on the Product under this Agreement and such cessation of work and any consequential delay to the Project Plan shall not amount to any breach of this Agreement;
  - (c) The Parties shall discuss how to deal with such Third Party claim.
- 14.6 If a solution is not found to resolve this Patent Claim within **[\*\*]** months of the date a Patent Claim is first received, then each Party shall be entitled to terminate this Agreement in accordance with Section 19.2(b).
- 14.7 Each Party shall be responsible for their own costs of dealing with and resolving any such Third Party Patent claim.

#### 15 Representations and Warranties

- 15.1 Institut Pasteur. Institut Pasteur hereby represents and warrants to AMT that:

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- (a) Materials and Information Supplied to AMT. Institut Pasteur is free to disclose to AMT Institut Pasteur Confidential Information and any other information or materials supplied by Institut Pasteur to AMT in accordance with this Agreement.
  - (b) No Patent Infringement Notice. At the Effective Date, no Third Party has filed and served on Institut Pasteur or any other Consortium Member, nor threatened in writing to the Institut Pasteur or any other Consortium Member to file, pursue or maintain any claim, lawsuit, charge, complaint or other action alleging infringement by the Institut Pasteur of a Third-Party Patent based on the manufacture, use, import, offer for sale or sale of the Product;

- (c) No Hazards. Institut Pasteur has made AMT aware of any hazards reasonably known to it as of the Effective Date involved in handling the Raw Materials, the Product, and any Wastes generated through performance of the validation and manufacturing activities contemplated hereunder;
- (d) License. Institut Pasteur has the right, power and authority to grant AMT the license set forth in Section 14.1 and will not during the term of this Agreement enter into any contract, arrangement or commitment in the future which prohibits the grant of such license.
- (e) Power and Authority. Institut Pasteur has the corporate power, the authority, and the legal right to enter into this Agreement and to perform its obligations under this Agreement; and
- (f) Execution, Delivery and Performance of the Agreement. Institut Pasteur has taken all necessary corporate action on its part to authorize the execution and delivery of this Agreement and the performance of its obligations under this Agreement. This Agreement has been duly executed and delivered on behalf of Institut Pasteur, and constitutes a legal, valid, binding obligation, enforceable against Institut Pasteur and its successors and assigns in accordance with its terms, except as enforceability may be limited by law. The execution, delivery and performance of this Agreement does not breach, violate, contravene or constitute a default by the Institut Pasteur under any contracts, arrangements or commitments to which Institut Pasteur is a party or by which it is bound nor does the execution, delivery and performance of this Agreement by Institut Pasteur violate any order, law or regulation of any court, governmental body or administrative or other agency having authority over it.

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15.2 AMT. AMT hereby represents, undertakes and warrants to Institut Pasteur that:

- (a) Materials and Information Supplied to Institut Pasteur. AMT is free to disclose to Institut Pasteur AMT Confidential Information and any other information or materials supplied by AMT to Institut Pasteur in accordance with this Agreement.
- (b) No Patent Infringement Notice. At the Effective Date, no Third Party has filed and served on AMT, nor threatened in writing to the AMT to file, pursue or maintain any claim, lawsuit, charge, complaint or other action alleging infringement by AMT or AMT Affiliates of a Third-Party Patent based on the manufacture, use, import, offer for sale or sale of the Product;
- (c) Product. AMT warrants the Product manufactured under this Agreement, and in particular that all Clinical Batches of Product manufactured hereunder: (i) shall be manufactured and analyzed in conformance with the Master Production Record and the Quality Agreement; (ii) shall be manufactured in compliance with the requirements of cGMP and all other applicable laws and regulations; (iii) shall be packaged in accordance with the Shipping Guidelines; (iv) shall be transferred free and clear of any liens or encumbrances of any kind to the extent arising through or as a result of the acts or omissions of AMT or its suppliers and (iv) shall conform, at the time of delivery, to the Release Specification.
- (d) Manufacturing. The Development, the Manufacturing Process and AMT's Quality Review and Approval shall be conducted in compliance with applicable cGMP and the Quality Agreement and all other applicable laws and regulations;
- (e) No Hazards. AMT has made Institut Pasteur aware of any hazards reasonably known to it as of the Effective Date involved in handling the Raw Materials, the Product, and any Wastes generated through performance of the validation and manufacturing activities contemplated hereunder;
- (f) License. AMT has the right, power and authority to grant Institut Pasteur the license set forth in Sections 14.2 and 14.3 and will not during the term of this Agreement enter into any contract, arrangement or commitment in the future which prohibits the grant of such license.
- (g) Power and Authority. AMT has the corporate power, authority and the legal right to enter into this Agreement and to perform its obligations under this Agreement;
- (h) Execution, Delivery and Performance of Agreement. AMT has taken all necessary corporate action on its part to authorize the execution and delivery of this Agreement and the performance of its obligations under this Agreement. This Agreement has been duly executed and delivered on

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behalf of AMT, and constitutes a legal, valid, binding obligation, enforceable against AMT in accordance with its terms except as enforceability may be limited by law. The execution, delivery and performance of this Agreement does not breach, violate, contravene or constitute a default by AMT under any contracts, arrangements or commitments to which AMT is a party or by which it is bound nor does the execution, delivery and performance of this Agreement by AMT violate any order, law or regulation of any court, governmental body or administrative or other agency having authority over it.

15.3 The Parties agree and acknowledge as follows:

- (a) that, in accordance with the provisions of this Agreement, AMT shall take a Reference Materials sample of each Clinical Batch manufactured under this Agreement upon completion of its manufacture, which sample shall be a representative sample of the Clinical Batch delivered to the Institut Pasteur (the "Delivery Sample");
- (b) that the Delivery Sample shall be retained at its own costs by AMT during the term of this Agreement and for a minimum period of [\*\*] months following its termination; and
- (c) that, in any circumstance of dispute between the Parties as to the conformance of the relevant Clinical Batch with the requirements of Section 15.2(c)(v) above, such Delivery Sample shall be made available by AMT for the purposes of testing (whether by the Expert in

accordance with Section 13.2 or otherwise) without unreasonable delay, and the Parties agree that (save in circumstances where the storage of the Delivery Sample has not been in accordance with the Storage Guidelines) the Conformance or Non-Conformance of such Delivery Sample shall, for the purposes of this Agreement, be deemed to be conclusive as to the Conformance or Non-Conformance of the Conformance or Commercial Batch from which it was taken.

- 15.4 Disclaimer. OTHER THAN AS SET FORTH IN SECTION 15, ALL OTHER WARRANTIES, BOTH EXPRESS AND IMPLIED, ARE HEREBY EXPRESSLY DISCLAIMED, INCLUDING, WITHOUT LIMITATION, ANY WARRANTY OF MERCHANTABILITY OR FITNESS FOR A PARTICULAR PURPOSE OF THE PRODUCT OR THE SERVICES PROVIDED HEREUNDER. OTHER THAN THE DEVELOPMENT AND MANUFACTURING SERVICES PROVIDED HEREUNDER, AMT HAS NOT PARTICIPATED IN THE RESEARCH AND DEVELOPMENT OF THE PRODUCT, HAS NOT IN ANY WAY EVALUATED THE PRODUCT'S OR PRODUCT'S SAFETY OR EFFICACY IN HUMANS OR OTHERS, AND SHALL NOT IN ANY WAY BE LIABLE FOR INSTITUT PASTEUR'S USE OF A CLINICAL BATCH WHICH HAS BEEN PRODUCED BY AMT IN ACCORDANCE WITH THE TERMS OF THIS AGREEMENT.

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## 16 Indemnification

- 16.1 AMT shall indemnify and hold harmless Institut Pasteur from and against any claims, losses, liabilities, costs (including, without limitation, reasonable attorneys' fees and expenses), damages and expenses arising out of or in connection with any claim by a Third Party arising out of:

- (a) any material breach by AMT of this Agreement;
- (b) AMT's grossly negligent acts or omissions or willful misconduct; and/or

provided that:

- (a) Institut Pasteur gives notice to AMT of such claim as soon as reasonably possible upon becoming aware of the same; and
- (b) Institut Pasteur gives AMT the sole conduct of the defence and settlement of such claim and does not at any time admit liability or otherwise attempt to settle the claim subject to AMT providing reasonable assurances, to Institut Pasteur's reasonable satisfaction, with respect to AMT's ability to pay for any costs or liabilities which Institut Pasteur may incur by reason of AMT's conduct of such defence or settlement of such claims; provided, however, that (i) any such settlement by AMT shall not adversely affect Institut Pasteur's rights under this Agreement or impose any obligations on Institut Pasteur in addition to those set forth herein, and (ii) Institut Pasteur shall have the right, but not the obligation, to be represented by counsel of its own selection and expense.

- 16.2 Subject to the provisions of the article 16.1 of this Agreement, Institut Pasteur shall indemnify and hold harmless AMT from and against any claims, losses, liabilities, costs (including, without limitation, reasonable attorneys' fees and expenses), damages and expenses arising out of or in connection with any claim by a Third Party arising out of:

- (a) any material breach by Institut Pasteur of this Agreement;
- (b) the packaging, testing, labelling, handling, distribution, use, import or sale of the Product in any form;
- (c) Institut Pasteur's grossly negligent acts or omissions or willful misconduct; and/or

provided that

- (a) AMT gives notice to Institut Pasteur of such claim as soon as reasonably possible upon becoming aware of the same; and

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- (b) AMT gives Institut Pasteur the sole conduct of the defence and settlement of such claim and does not at any time admit liability or otherwise attempt to settle the claim subject to Institut Pasteur providing reasonable assurances, to AMT's reasonable satisfaction, with respect to Institut Pasteur's ability to pay for any costs or liabilities which AMT may incur by reason of Institut Pasteur's conduct of such defence or settlement of such claims; provided, however, that (i) any such settlement by Institut Pasteur shall not adversely affect AMT's rights under this Agreement or impose any obligations on AMT in addition to those set forth herein, and (ii) AMT shall have the right, but not the obligation, to be represented by counsel of its own selection and expense.

- 16.3 Insurance. AMT shall maintain public liability insurance with an indemnity limit of [\*\*] Euros (€[\*\*]) for any one occurrence. AMT shall maintain appropriate commercial general liability insurance including, without limitation, product liability and contractual liability coverage with respect to the development, manufacture, import, sale, offer for sale and use of the Manufacturing Process, Deliverables and Product in an amount equal to [\*\*] Euros (€[\*\*]). AMT shall maintain such insurance for so long as it continues to manufacture or sell Product or services and for a period of [\*\*] years after the end of this Agreement.

- 16.4 Disclaimer of Consequential Damages. IN NO EVENT SHALL EITHER PARTY BE LIABLE TO THE OTHER OR ANY OF ITS AFFILIATES FOR ANY CONSEQUENTIAL, INCIDENTAL, INDIRECT, SPECIAL, PUNITIVE OR EXEMPLARY DAMAGES (INCLUDING, WITHOUT LIMITATION, LOST PROFITS, BUSINESS OR GOODWILL) SUFFERED OR INCURRED BY SUCH OTHER PARTY OR ITS AFFILIATES IN CONNECTION WITH THIS AGREEMENT, EVEN IF ADVISED OF THE POSSIBILITY OF SUCH DAMAGES.

- 16.5 Limitation of Liability. BOTH PARTIES HEREBY AGREE THAT TO THE FULLEST EXTENT PERMITTED BY LAW, AMT'S LIABILITY TO INSTITUT PASTEUR, FOR ANY AND ALL INJURIES, CLAIMS, LOSSES, EXPENSES, OR DAMAGES, WHATSOEVER, ARISING OUT OF OR IN ANY WAY RELATED TO THIS AGREEMENT FROM ANY CAUSE, INCLUDING, BUT NOT LIMITED TO, NEGLIGENCE, ERRORS, OMISSIONS OR STRICT LIABILITY, SHALL NOT EXCEED THE AMOUNT PAID TO AMT

UNDER THIS AGREEMENT. TO THE EXTENT THAT THIS CLAUSE CONFLICTS WITH ANY OTHER CLAUSE, THIS CLAUSE SHALL TAKE PRECEDENCE OVER SUCH CONFLICTING CLAUSE. IF APPLICABLE LAW PREVENTS ENFORCEMENT OF THIS CLAUSE, THEN THIS CLAUSE SHALL BE DEEMED MODIFIED TO PROVIDE THE MAXIMUM PROTECTION FOR AMT AS IS ALLOWABLE UNDER APPLICABLE LAW. THE FOREGOING LIMITATION SHALL NOT APPLY TO LIABILITY ARISING FROM DEATH OR PERSONAL INJURY DIRECTLY CAUSED BY AMT'S NEGLIGENCE OR FROM FRAUDULENT ACTS OR WILFUL MISCONDUCT.

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17 Confidentiality

- 17.1 AMT Confidentiality Obligations. AMT shall not use Institut Pasteur Confidential Information except as authorized under this Agreement and shall not disclose Institut Pasteur Confidential Information to any Third Party other than: (i) employees, consultants, agents or Subcontractors of AMT or AMT's Affiliates who are bound by similar obligations of confidentiality and nonuse and who have a need to know such information in order to perform their duties or services in connection with AMT's obligations under this Agreement; (ii) any Regulatory Authority to the extent that it requires such information in connection with making Regulatory Filings and maintaining Regulatory Authority approvals for the Product under the provisions of this Agreement and on Institut Pasteur's request; or (iii) any Governmental Authority in connection with securing and/or maintaining registrations under the provisions of this Agreement and on Institut Pasteur's request.
- 17.2 Institut Pasteur Confidentiality Obligations. Institut Pasteur shall not use AMT Confidential Information except as authorized under this Agreement and shall not disclose any AMT Confidential Information to any Third Party other than as authorized under this Agreement and other than : (i) employees, consultants, or agents of Institut Pasteur who are bound by similar obligations of confidentiality and nonuse and who have a need to know such information in order to perform their duties or services in connection with Institut Pasteur's obligations under this Agreement or the development or commercialization of the Product; (ii) any Regulatory Authority to the extent that it requires such information in connection with making Regulatory Filings and maintaining Regulatory Authority approvals for the Product or (iii) any Governmental Authority in connection with securing and/or maintaining registrations.
- 17.3 Responsibility for Compliance with Confidentiality and Nonuse Obligations. Each Party shall be responsible for any intentional misuse or misappropriation, by such Party, its Affiliates, or the employees, consultants, agents, Subcontractors or sublicensees of such Party or such Party's Affiliates, of the other Party's Confidential Information. Each Party shall use its reasonable endeavours to enforce the obligations of confidence imposed upon such persons by it in accordance with Section 17.8.
- 17.4 Terms of Agreement. Except for any disclosure that is necessary to comply with national rules or regulations (including the rules and regulations of any national stock exchange on which such Party's securities are traded) or disclosure to a Party's employees, Affiliates, consultants, agents, professional advisers, Subcontractors, sublicensees, potential acquirors, investors or potential investors under similar conditions of confidentiality, or to the extent necessary in order to enforce its rights in any court of competent jurisdiction or in any arbitration proceedings or in order to participate in any such proceedings neither Party shall, without the prior written consent of the other Party which consent may be dependant on the disclosure being under similar conditions of confidentiality to this

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Agreement, disclose in any manner to any Third Party the terms and conditions of this Agreement.

- 17.5 Exclusions. The obligations of confidentiality and nonuse set forth in Section 17.1 and Section 17.2 shall not apply to any information for which it is evidenced that: (i) at the time of disclosure, is known publicly or thereafter becomes known publicly through no fault of the recipient, its Affiliates, their employees, consultants, agents, Subcontractors or sublicensees; (ii) becomes available to the recipient from a Third Party which is not legally prohibited from disclosing such information; (iii) was developed by the recipient independently of Confidential Information obtained from the disclosing Party as evidenced by written records; (iv) was already known to the recipient before receipt from the disclosing Party, as shown by its prior written records; or (v) is released with the prior written consent of the disclosing Party. In determining whether or not the disclosing Party's Confidential Information has entered the public domain, the obligations of confidentiality shall no longer apply to only that portion of said Confidential Information that has become public, and portions remaining confidential shall retain their status as Confidential Information.
- 17.6 Notification of Mandatory Disclosure.
- (a) Notification and Consultation. In the event that a Party (in such case, the "Notifying Party") believes it is required by applicable statute or regulation (including the rules and regulations of any national stock exchange on which such Party's securities are traded), or by judicial or administrative process to disclose any part of the other Party's (in such case, the "Notified Party") Confidential Information, the Notifying Party shall (1) promptly notify the Notified Party of each such requirement and identify the documents so required to be disclosed thereby, so that the Notified Party may seek an appropriate protective order or other remedy and/or waive compliance by the Notifying Party with the provisions of this Agreement, and (2) consult with the Notified Party on the advisability of taking legally available steps to resist or narrow the scope of such disclosure.
- (b) Limited Disclosure. If, in the absence of such a protective order or such a waiver by the Notified Party of the provisions of this Agreement, the Notifying Party is nonetheless required by applicable law to disclose any part of the Notified Party's Confidential Information, the Notifying Party may disclose such Confidential Information without liability under this Agreement, except that the Notifying Party shall furnish only that portion of the Confidential Information which is legally required to be disclosed.
- 17.7 No Licenses. Except as expressly provided in Section 14 hereof, no right or license, either express or implied, is granted under any Intellectual Property right or by virtue of the disclosure of Confidential Information under this Agreement, or otherwise.

- 17.8 Maintenance of Confidentiality. Each Party shall apply at least the same level of security to the other Party's Confidential Information as it would to its own most confidential information and shall use all reasonable and customary precautions to safeguard the confidentiality of the other Party's Confidential Information, including ensuring that all employees, consultants, agents, Subcontractors or sublicensees who are provided access to such Confidential Information are informed of the confidential and proprietary nature of such Confidential Information and are subject to enforceable contractual confidentiality and nonuse obligations that are at least as restrictive as those contained in this Agreement.
- 17.9 Equitable Relief. Each Party agrees that (i) the other Party and its Affiliates would be irreparably injured by a material breach of the confidentiality and nonuse provisions of this Agreement by the employees, consultants, agents, Subcontractors or sublicensees of the breaching Party or its Affiliates, (ii) that monetary remedies would be inadequate to protect the other Party against any actual or threatened material breach of the provisions of this Section 17 by the employees, consultants, agents, Subcontractors or sublicensees of the breaching Party or its Affiliates, and, (iii) without prejudice to any other rights and remedies otherwise available to the other Party, the breaching Party agrees, upon proof of any such actual or threatened material breach, to the granting of equitable relief, including injunctive relief and specific performance, in the other Party's favor.
- 17.10 Duration. The obligation of confidentiality and non use of this Section 17 shall be effective during this Agreement and for [\*\*] years after the expiration or termination of this Agreement save in respect of any Manufacturing Documentation, the Master Production Record or any other information relating to the Manufacturing Process for which the obligations of confidentiality and non use under this Section 17 shall continue without limit in time.
- 18 Use of Names
- Neither Party shall make use of the name of the other Party in any advertising or promotional material, or otherwise, in connection with this Agreement or any related agreements, without the prior written consent of such other Party.
- To the extent useful or required for disclosure purposes AMT will make a public announcement about the relationship with Institut Pasteur. The language will be subject to the prior written approval of Institut Pasteur.
- 19 Term; Termination
- 19.1 Term; Option to Extend. Unless sooner terminated pursuant to the terms of this Agreement, the term of this Agreement shall commence on the Effective Date and shall continue until the completion of the Parties' obligations under this Agreement, provided however that the delivery of the Clinical Batch shall be effective twelve (12) months after the Effective Date. However, in case of delay due to scientific, technological or regulatory problems, the Parties will meet to

analyse them and determine together an additional reasonable extension period necessary to solve the problem concerned.

- 19.2 Termination. This Agreement may be terminated as follows:
- (a) AMT may immediately terminate this Agreement after provision of the First Clinical Batch, by notifying thirty (30) days in advance Institut Pasteur in writing that it will not manufacture the Second Clinical Batch.
  - (b) If a solution is not found to resolve a Patent Claim within [\*\*] months of the date a Patent Claim is first received in accordance with Section 14.6, then each Party shall be entitled to terminate this Agreement immediately by providing notice to the other Party.
  - (c) Termination on Notice. Each Party may terminate this Agreement, without liability to the other Party, by giving thirty (30) days notice to the other Party if it reasonably believes that there are serious issues with respect to the Product or to the Second Batch, such that the continuation of the Agreement is not possible for technical feasibility reasons (including vector specifications).
  - (d) Termination by Institut Pasteur. Institut Pasteur may terminate this Agreement by giving thirty (30) days notice to AMT in the circumstances set out in Sections 6.3 and 13.4 or in case of refusal by the regulatory authority to perform the clinical trial, subject in this last case to the payment of the services performed by AMT.
  - (e) Material Breach. Either Party may terminate this Agreement, by written notice to the other Party, for any material breach of this Agreement by the other Party, if such breach is not cured within [\*\*] days after the breaching Party receives written notice of such breach from the nonbreaching Party; provided, however, if such breach is not capable of being cured within such [\*\*] day period, the cure period shall be extended on a mutual reasonable consent for such amount of time as may be reasonably necessary to cure such breach, so long as the breaching Party is making diligent efforts to do so. Such termination shall be effective upon expiration of such cure period.
  - (f) Force Majeure; No-Fault Termination. Either Party shall have the right to terminate this Agreement, without liability to the other Party, upon providing written notice thereof to the other Party, such termination to be effective thirty (30) days from the date of such notice if, as a result of a Force Majeure Event, a Party is unable fully to perform its obligations under this Agreement for any continuous period of one hundred and eighty (180) days.
  - (g) Insolvency. Either Party may terminate this Agreement upon notice to the other Party, upon (a) the dissolution, termination of existence, liquidation or business failure of the other Party; (b) the appointment of a custodian or

receiver for the other Party who has not been terminated or dismissed within ninety (90) days of such appointment; (c) the institution by the other Party of any proceeding under national bankruptcy, reorganization, receivership or other similar laws affecting the rights of creditors generally or the making by such Party of a composition or any assignment for the benefit of creditors under any national

bankruptcy, reorganization, receivership or other similar law affecting the rights of creditors generally, which proceeding is not dismissed within ninety (90) days of filing.

- (h) Cumulative Remedies. Any right to terminate this Agreement shall be in addition to and not in lieu of all other rights or remedies that the Party giving notice of termination may have at law or in equity or otherwise.

### 19.3 Consequences of Termination.

- (a) Payment of Amounts Due. Expiration or termination of this Agreement for any reason shall not exempt, unless otherwise agreed between the Parties in this Agreement, any Party from paying to the other Party any amounts properly due but unpaid to such Party at the time of such expiration or termination, including, without limitation, payments due under Section 8 hereof, on a prorata basis according to the work carried out up to that date, except in case of termination for AMT's breach.
- (b) Termination of Development Activities. The Parties shall mutually agree in good faith and as soon as reasonably possible upon which Development milestone activities that are in progress as of the effective date of any termination hereunder shall be completed and which shall be terminated effective immediately. The Agreement shall continue to survive with respect to those in progress milestone activities which the Parties agree to continue in good faith.
- (c) Termination of Runs. Runs that are in process as of the effective date of any termination hereunder shall not be cancelled unless otherwise agreed by the Parties in writing and the Agreement shall continue to survive with respect to those in process Runs. Product that has been fully manufactured as of the date of such termination, but for which Quality Review and Approval has not been completed, shall remain subject to the terms of this Agreement, and the Agreement shall continue to survive with respect to such Product, unless otherwise agreed by the Parties.
- (d) Raw Materials, Consumables and Resins. Upon expiration or termination of this Agreement Institut Pasteur shall purchase from AMT (to the extent not previously paid for by Institut Pasteur), at AMT's Acquisition Cost, all remaining usable Raw Materials and Consumables acquired and paid for by AMT for the manufacture of Product under this Agreement, provided however, that as of the date of receipt of the termination notice, AMT shall place no further orders for Raw Materials and Consumables except as may

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be necessary for completion of any portion of AMT's services hereunder that are not immediately terminated, according to Sections 19.3 (b) and (c).

- (e) Return of Materials, of Institut Pasteur Confidential Information and Institut Pasteur Background Information. Upon expiration or termination of this Agreement, unless otherwise directed by Institut Pasteur, AMT shall promptly and at Institut Pasteur's sole cost and expense (except in case of termination for AMT's breach) deliver to Institut Pasteur or, at Institut Pasteur's election, destroy, (i) all Institut Pasteur Confidential Information and Institut Pasteur Background Information, except for a single copy and/or sample which may be retained to record its obligations under this Agreement only and which shall remain subject to the obligations of nonuse and confidentiality set forth in this Agreement, (ii) all Reference Materials being held by AMT (except that AMT shall have the right to keep a retained sample of each Reference Material according to the provisions of this Agreement), and (iv) all remaining Raw Materials, Consumables purchased pursuant to Section 19.3(d), (iii) all work in progress, (iv) all completed and in-progress Batches, (v) all Batch Disposition Documentation to the extent not previously provided and (vi) all other items in the possession, power or control of AMT, title to which is (or is to be, upon due delivery and payment therefor under this Agreement) held by Institut Pasteur. AMT shall in no way be responsible for any claims, demands, losses, liabilities, expenses or damages, whatsoever, arising out of or in anyway related to Institut Pasteur's use of any such "work in progress" or "in progress Batches" delivered up. If any Institut Pasteur owned property (Raw Materials, Product etc.) remains at the AMT Facility for a period longer than six (6) months after expiration or termination of this Agreement, Institut Pasteur shall pay for such storage as Additional Services.
- (f) Return of AMT Confidential Information. Upon expiration or termination of this Agreement, Institut Pasteur shall promptly return all AMT Confidential Information to AMT, except for a single copy which may be retained for documentation purposes only and which shall remain subject to the obligations of nonuse and confidentiality set forth in this Agreement.
- (g) Grant of Licence to Institut Pasteur. In the event of termination by AMT in accordance with Clause 19.2(a) AMT shall grant Institut Pasteur the licence set out in, and in accordance with, Sections 11.8 to 11.11 and 14.3.
- (h) Accrued Rights. Except as otherwise expressly set forth herein, any termination or expiration of this Agreement shall be without prejudice to any right which shall have accrued to the benefit of either Party and shall not relieve either Party of any obligation which has accrued prior to the effective date of such termination or expiration, which obligations shall remain in full force and effect for the period provided therein or, if no period is provided therein, then such obligations shall remain in full force and effect indefinitely.

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- 19.4 Surviving Rights. Sections 1, 2.5, 9, 10, 11, 14 to 19, 21 and any other terms of this Agreement (to the extent they are intended to survive the termination or expiration of this Agreement), together with the rights and obligations contained therein, shall survive the termination or expiration of this Agreement.

## 20 Force Majeure

- 20.1 Effects of Force Majeure. No Party shall be in breach of this Agreement if there is any failure of performance under this Agreement (except for payment of any amounts due under this Agreement) occasioned by any reason beyond the control and without the fault or negligence of the Party affected thereby, including, without limitation, an act of God, fire, flood, act of government or state, war, civil commotion, insurrection, acts of terrorism, embargo, sabotage, or any other reason beyond the control and without the fault or negligence of the Party affected thereby (a "Force Majeure Event"). Such excuse shall continue as long as the Force Majeure Event continues. Upon cessation of such Force Majeure



Event, the affected Party shall promptly resume performance under this Agreement as soon as it is commercially reasonable for the Party to do so.

- 20.2 **Notice of Force Majeure.** Each Party agrees to give the other Party prompt written notice of the occurrence of any Force Majeure Event, the nature thereof, and the extent to which the affected Party will be unable to fully perform its obligations under this Agreement. Each Party further agrees to use commercially reasonable efforts to correct the Force Majeure Event as quickly as practicable (provided that in no event shall a Party be required to settle any Labor dispute) and to give the other Party prompt written notice when it is again fully able to perform such obligations.
- 20.3 **Termination.** This Agreement may be terminated as a result of a Force Majeure Event only in accordance with Section 19.2(e) hereof.

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## 21 Miscellaneous

- 21.1 **Notices.** Any notice required or permitted to be given under this Agreement by any Party shall be in writing and shall be (a) delivered personally, (b) sent by registered mail, return receipt requested, postage prepaid, (c) sent by a nationally-recognized courier service guaranteeing next-Working Day or second Working Day delivery, charges prepaid, or (d) delivered by facsimile (with documented evidence of transmission), to the addresses or facsimile numbers of the other Party set forth below, or at such other addresses as may from time to time be furnished by similar notice by any Party. The effective date of any notice under this Agreement shall be the date of receipt by the receiving Party.

If to AMT:

For the Attention of the Chief Executive Officer  
Amsterdam Molecular Therapeutics (AMT) B.V.  
Meibergdreef 61  
1105BA Amsterdam  
The Netherlands

If to Institut Pasteur:

For the attention of the Legal Director  
25-28, rue du Docteur Roux 75724 Paris Cedex 15, France

And

For the attention of the Medical Director  
25-28, rue du Docteur Roux 75724 Paris Cedex 15, France

- 21.2 **Applicable Law.** This Agreement shall be construed, interpreted and enforced in accordance with the internal substantive laws of the Netherlands, without reference to the choice of law doctrine of such state.
- 21.3 **Headings.** All headings in this Agreement are for convenience of reference only and shall not affect the interpretation of this Agreement.
- 21.4 **Exhibits.** All exhibits or appendices referred to herein form an integral part of this Agreement and are incorporated into this Agreement by such reference.
- 21.5 **Security Procedures.** All Institut Pasteur personnel visiting or having access to the AMT Facility agree to abide by AMT standard policies, operating procedures and security procedures as established by AMT and communicated to Institut Pasteur.
- 21.6 **Assignment.** This Agreement shall be binding upon the successors and assigns of the Parties and the name of a Party appearing herein shall be deemed to include the names of its successors and assigns. Neither Party may assign its interest under this Agreement without the prior written consent of the other Party, such consent not to be unreasonably withheld; **provided, however**, either Party may assign its interest under this Agreement, without the prior written consent of the other Party to an

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Affiliate but only for as long as such Affiliate remains an Affiliate of the relevant Party. Any permitted assignment of this Agreement by either Party will be conditioned upon that Party's permitted assignee agreeing in writing to comply with all the terms and conditions contained in this Agreement. Any purported assignment without a required consent shall be void. No assignment shall relieve any Party of responsibility for the performance of any obligation that accrued prior to the effective date of such assignment.

- 21.7 **Severability.** If any part of this Agreement shall be found to be invalid or unenforceable under applicable law in any jurisdiction, such part shall be ineffective only to the extent of such invalidity or unenforceability in such jurisdiction, without in any way affecting the remaining parts of this Agreement in that jurisdiction or the validity or enforceability of the Agreement as a whole in any other jurisdiction. In addition, the part that is ineffective shall be reformed in a mutually agreeable manner so as to as nearly approximate the intent of the Parties as possible.
- 21.8 **Independent Contractors.** Each of the Parties is an independent contractor and nothing herein contained shall be deemed to constitute the relationship of partners, joint venturers, nor of principal and agent between the Parties. Neither Party shall at any time enter into, incur, or hold itself out to Third Parties as having authority to enter into or incur, on behalf of the other Party, any commitment, expense, or liability whatsoever.
- 21.9 **Waiver.** No waiver of any term, provision or condition of this Agreement whether by conduct or otherwise in any one or more instances shall be deemed to be or construed as a further or continuing waiver of any such term, provision or condition or of any other term, provision or condition of this Agreement.

- 21.10 Counterparts. This Agreement and any amendment hereto may be executed in any number of counterparts, each of which shall for all purposes be deemed an original and all of which shall constitute the same instrument. This Agreement shall be effective upon full execution by facsimile or original, and a facsimile signature shall be deemed to be and shall be as effective as an original signature.
- 21.11 No Solicitation of Employees. During the Term and for one (1) years thereafter, each of the Parties agrees not to seek to induce or solicit any employee of the other Party or its Affiliates to discontinue his or her employment with the other Party or such Affiliate in order to become an employee or an independent contractor of the soliciting Party or its Affiliates; provided, however, that neither Party shall be in violation of this Section 21.11 as a result of making a general solicitation for employees or independent contractors. For the avoidance of doubt the publication of an advertisement shall not constitute solicitation or inducement.
- 21.12 Entirety; Amendments. This Agreement, including any exhibits attached hereto and referenced herein, constitutes the full understanding of the Parties and a complete and exclusive statement of the terms of their agreement with respect to

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the specific subject matter hereof, and no terms, conditions, understandings or agreements purporting to modify or vary the terms thereof shall be binding unless hereafter made in a written instrument referencing this Agreement and signed by each of the Parties.

- 21.13 Preamble. The Preamble and the Background referred to herein form an integral part of this Agreement.
- 21.14 Preference. The terms of this Agreement shall prevail in the event of a conflict between this Agreement and any exhibits or appendices.

[the remainder of this page intentionally blank]

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IN WITNESS WHEREOF, the Parties have caused this Agreement to be executed as of the Effective Date.

INSTITUT PASTEUR

By: /s/ A. Douly  
 Name: \_\_\_\_\_  
 Title: \_\_\_\_\_  
 Date: January 7<sup>th</sup>, 2011

AMSTERDAM MOLECULAR THERAPEUTICS (AMT) BV

By: /s/ J. Alday  
 Name: \_\_\_\_\_  
 Title: \_\_\_\_\_  
 Date: 11 01 2011

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#### Exhibit A Project Plan

Sanfilippo B or mucopolysaccharidosis type IIIB (MPSIIIB) is a lysosomal storage disease for which currently no treatment is available. The rationale for the present approach is the observation that the phenotype of MPSs can be reversed by enzyme replacement therapy. In case of Sanfilippo B the disease is caused by an autosomal recessive genetic defect of the lysosomal enzyme  $\alpha$ -N-acetylglucosaminidase (NaGlu). It was shown recently in different preclinical models that gene delivery of NaGlu has the potential to cure Sanfilippo B. The goal of the present project is to produce GMP grade AAV-NaGlu vector in sufficient amounts for clinical trials. The efficacy and safety of this vector will be tested in different animal models (for efficacy by measuring enzyme activity in MPSIIIB mice, for safety by performing toxicology and biodistribution studies in normal rats and dogs) before it will be applied in a phase I clinical trial.

#### Responsibilities

The table below outlines the main tasks and the parties responsibility for the delivery of the task.

	Task	AMT responsibility	Institut Pasteur responsibility
1	Process Development	[**]	[**]
2	Assay Development	[**]	[**]
3	<i>In vivo</i> tests in mice	[**]	[**]
	<i>Tox in rats and dogs</i>		[**]
4	MSV/WSV for cGMP batches	[**]	[**]
5	cGMP batch production	[**]	[**]

Assay to be developed

AAV5-specific assays will be developed by AMT; these will include:

[\*\*]

In addition, AMT will develop a transgene specific Q-PCR. A basic protocol has been already established at AMT, however, this process needs to be qualified prior to the initiation of the clinical phase I study.

A second Q-PCR will be established in collaboration with a service provider. This Q.-PCR method will be used by Institut Pasteur in biodistribution studies in rats.

Develop production platform

AMT will develop a robust upstream and downstream process suitable for the production of GMP material. The progress of the development process will be monitored by Q-PCR (and in vivo studies in mice). The in vivo studies will measure the transduction efficiency of AAV5-NaGlu, and will be used as potency assay. Institut Pasteur will be responsible for the in vivo potency assays that will accompany the process development work. MPSIIIB mice will be used for the

study. The mice will be diagnosed at birth by PCR, they will be used for the study when aged between week [\*\*]. The material from the [\*\*] development batches will be used to perform these studies. The NaGlu catalytic activity resulting from gene transfer by the AAV5-NaGlu vector will be measured in brain extracts approximately [\*\*] weeks post injection.

AMT has planned to deliver a maximum of [\*\*] development batches during this phase. The production of the [\*\*] development batch will be done adapting the AAV5-NaGlu production process previously developed at AMT.

The current AAV5 process is based on AMT’s proprietary baculovirus / insect cell platform which will be used to produce the AAV5-NaGlu vector. The vector will be purified by DNA-ase incubation followed by a chemical lysis step after which cell debris is removed by depth filtration. The clarified harvest is incubated overnight as a viral inactivation step. The inactivated material is purified by affinity chromatography and ion-exchange chromatography after which buffer exchange is performed using ultrafiltration/diafiltration (UF/DF). The intended formulation buffer consists of phosphate buffered saline (PBS) supplemented with [\*\*]. After UF/DF the product is frozen at [\*\*] and samples are analyzed according to a predetermined list of analyses and specification. The result of the Q-PCR analysis will be used to dilute the product to the target concentration of the finished product. The product will be filled into [\*\*] vials with a chorobutyl stopper and aluminum cap filled with [\*\*] of finished product. The closed vials will be stored at [\*\*] for storage until release and use.

Following the delivery of the batch, Institut Pasteur will test the batch in vivo in a mouse model (in vivo potency study). If needed, further process optimization will be employed for the production of the [\*\*] development batch, which will be again tested as above. Final process optimization work will result in the production of the [\*\*] development batch, which will be again tested as above. The process used for the most successful in vivo study (highest NaGlu activity per mg of protein in extracts prepared from the entire injected brain hemisphere) will be employed for the production of the Tox and clinical material. Purchasing new equipment for optimization of the manufacturing process is not included in the scope of this project.

Project phases

1. Development batches

AMT will deliver maximally [\*\*] development batches which will be performed on a maximum of [\*\*] scale. The purified product will be tested with a Q-PCR by AMT to determine the strength. A sample will be shipped to Institut Pasteur for an in vivo test in the mouse model to test its in vivo activity and whether it meets the criteria (to be established) for further product development. After completion of each batches there will be a formal Go/No Go based on the outcome of the in-vivo test results obtained by Institut Pasteur. The in vivo tests determine whether the product produced by AMT would be meeting the requirements for a clinical study. AMT and Institut Pasteur have to define the requirements for successful in vivo activity.

2. Analytical development

AMT will develop some assays for the AAV5-NaGlu vector characterization. These will include:

[\*\*]

For other assays such as general test for contaminations, sucrose content, bioburden, sterility, infectious particles assay, rcAAV, residual host cell DNA and protein, residual Triton, residual Benzonase it is assumed that no development is needed and this is therefore not included in the scope of this project.

A typical bill of testing for the finished product is listed in the Table below. Some process related impurities such as residual Benzonase, Triton, infectious baculovirus are tested on the active substance level and safety assays such as adventitious virus agents are tested during the manufacturing process on the bulk harvest prior to addition of Triton. AMT and Institut Pasteur have to agree on the number of (critical) in process controls for which AMT will make a proposal for discussion.

Tentative Acceptance Criteria - Batch 1

Test parameter	Tentative Acceptance Criteria
----------------	-------------------------------

[illegible]

***	***
***	***
	***
***	***
***	***

### 3. MSV/WSV production

#### 4. GMP batch

## 5. Stability study

## 6. Reference standard

## 8. Phase I/II clinical study

up to [\*\*] hours without charge.

9. **Viral clearance / inactivation study**

AMT will produce [\*\*] using an identical recipe to the cGMP clinical batch to generate materials for an adventitious viral agents removal study and for a baculovirus removal study. The [\*\*] study will be outsourced to a qualified contract laboratory. In the [\*\*] study one enveloped and one unenveloped model virus will be tested for inactivation and clearance by the process (singular experiment). AMT will report the first study and will make the reports available for submission of the IMPD or equivalent application for the clinical study by Institut Pasteur.

**Regulatory Affairs Strategy**

Contacts to AFSSAPS have already been established by Institut Pasteur. Institut Pasteur will be responsible for the interaction with AFSSAPS. Institut Pasteur will keep AMT informed about any advice received that relates to manufacturing.

Institut Pasteur is responsible for the orphan drug application. AMT will contribute information related to product development and manufacturing as part of this project, if any.

**QP Release**

AMT, as contract manufacturer, will release the manufacturing data as well as the quality control data generated by AMT or AMT’s vendors. The QP of AMT will provide Institut Pasteur with a certificate stating that production has taken place according to GMP and test results (excluding the potency assay carried out by Institut Pasteur below) comply with the specification.

Institut Pasteur is responsible for the evaluation of the potency assay. The QP of Institut Pasteur will release the product for clinical use, taking into account the QP release of AMT and the result of the potency assay.

**Project Plan - Delivery Dates**

[\*\*]

Milestones based on start of practical activities on [\*\*]:

[**]	[**]
[**]	[**]
[**]	[**]
[**]	[**]

Stability study: proposal for criteria for [\*\*] month study at timepoints [\*\*]

**Stability acceptance criteria for alipogene tiparvovec**

Test parameter	Acceptance Criteria	Method
General tests		
[**]	[**]	[**]
[**]	[**]	[**]
[**]	[**]	[**]
	[**]	
[**]	[**]	[**]
[**]	[**]	[**]
[**]	[**]	[**]
	[**]	
[**]	[**]	[**]
	[**]	
[**]	[**]	[**]
[**]	[**]	[**]
[**]	[**]	[**]
	[**]	
[**]	[**]	[**]
[**]	[**]	[**]
	[**]	

[**]	[**]	[**]
------	------	------

The estimate costs for development and production of the clinical batch(es) and licensing costs are in euros and are:

	Batch [**]	Batch [**]
<b>Development (including Quality Agreement):</b>		
Process development	[**]	
Assay development	[**]	
Virus removal study	[**]	
Stability study	[**]	[**]
Preliminary ref. standard qualification		
[**] GMP batch	[**]	
<b>Manufacture:</b>		
[**] GMP batch	[**]	[**]
IMPD / IB	[**]	
Qualification of analytics	[**]	[**]
Overheads on FTE	[**]	
<b>Total Cost estimate</b>	[**]	[**]

Discount

AMT recognise the substantial time committed to this project to date by all parties. In the interests of trying to support this project going forward, AMT is prepared to offer [\*\*] against the costs of each of Batch [\*\*] and Batch [\*\*] above.

Payment Plan

Signature fee	[**]
[**]	[**]
[**]	[**]
[**]	[**]
[**]	[**]
[**]	[**]

Additional Services

Additional storage of Batches, or any other Institut Pasteur owned property (Raw Materials, Product, etc) beyond the first [\*\*] month period : €[\*\*] per month per [\*\*] volume (or part thereof)

Provision of documents or other work product (other than the MPR, the Manufacturing Documentation and copies of Batch Disposition Documentation and any other documents or work product expressly required to be delivered under this Agreement) that do not exist as of the date of request or other substantive requests for assistance in compiling any Regulatory Filing : €[\*\*]/hour for AMT staff together with reimbursement of third party expenses as invoiced to AMT.

Additional manufacturing audits : €[\*\*]/hour for AMT staff together with reimbursement of third party expenses as invoiced to AMT.

Exhibit B

AMT’s Standard Formats for its Batch Disposition Documents

The Certificate of Analysis (CoA) shall be substantially in the form set out below:

[the remainder of this page intentionally blank]



# Certificate of Analysis

SMS number :  
Product :  
Part number :  
Batch number :  
Production stage :  
Expiry date :

Page 1 of 1

Test, Method	Specification	Result
pH, (QC-AIM-0013)		
Genome copies, (QC-AIM-0009)		
Endotoxin		
Osmolality, (QC-AIM-0017)		
Extractable Volume, (QC-AIM-0037)		
Color, (QC-AIM-0006)		
Clarity, (QC-AIM-0042)		
Sub-visible Particles, (Ph.Eur.2.9.19)		
Sub-visible Particles, (Ph.Eur.2.9.19)		
Residual Baculovirus DNA, (QC-AIM-0024)		
Residual SF+ DNA		
Residual SF+ Protein		
Sterility, (58201)		
Physical state, (QC-AIM-0008)		
Capsid protein composition by microfluidic electrophoresis, (QC-AIM-0031)		
Protein purity by micro fluidic electrophoresis, (QC-AIM-0031)		
Protein impurities by microfluidic electrophoresis, (QC-AIM-0031)		
Total Proteins, (QC-AIM-0022)		
Potency,		
Sucrose by refraction index, (QC-AIM-0008)		
Ratio full : infectious particles (gcp), (calculation)		
Ratio total: full particles (tp/gc), (QC-AIM-0025)		
Monomer AAV particles by DLS, (QC-AIM-0028)		
Particle aggregate by DLS, (QC-AIM-0028)		

Remarks : N/a

Conclusion : The results comply with the specifications.

Authorized signature : \_\_\_\_\_

Name : \_\_\_\_\_

AMT

AMT BV

Visiting address  
Meibergdreef 61  
1105 SA, Amsterdam  
The Netherlands

Postal address  
P.O. Box 22506  
1100 CA, Amsterdam  
The Netherlands

Tel +31 (0)20 566 7264  
Fax +31 (0)20 566 9272  
info@amtbioharma.com  
www.amtbioharma.com

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The Certificate of Release (CoR) shall be substantially in the form set out below:

[the remainder of this page intentionally blank]



## Certificate of Release

For product for clinical use

Product name:		Date manufactured:	
Proper name:		Expiry/retest date:	
Quantity:		Storage conditions:	
Batch number:			

Manufacturer:	AMT
Production Site:	Meibergdreef 61, 1105 BA Amsterdam, The Netherlands
Sponsor:	
Sponsor Address:	
Clinical Investigation Site (s):	
Address:	

### Release tests:

- ☐ : All test results are within approved specifications.
- ☐ : Not all testing specifications have been met. The rationale for use is appended.

### Certification statement:

I hereby certify that the above information is authentic and accurate. This batch has been manufactured at the above-mentioned site in full compliance with the EU GMP requirements and the specifications described in module 3 of the Investigational Medicinal Product Dossier (IMPD), date XXX, version X. The batch manufacturing and analytical records were reviewed and found to be in compliance with GMP.

AMT is certified by the Dutch Health Authorities (ministerie van VWS), manufacture licence no. 108990F, to manufacture biological products (gene therapeutics) for clinical trial use.

Name:	A. Vroege
Position:	Qualified Person
Signature:	
Date:	

### Release statement by Sponsor:



Considering that all requirements of article 9 of EU Directive 2001/20/EC have been fulfilled, I, as representative of the Sponsor, authorize the shipment of this batch of XXXX to the clinical investigation site(s) mentioned above.	
Name:	
Position:	
Signature:	
Date:	

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**Exhibit C**  
Quality agreement

To be completed within [\*\*] months after the Effective Date

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\_\_\_\_\_  
INSTITUT PASTEUR

(1)

and

AMSTERDAM MOLECULAR THERAPEUTICS (AMT) BV

(2)

\_\_\_\_\_  
AMENDMENT N°1 TO THE DEVELOPMENT  
and MANUFACTURING AGREEMENT  
\_\_\_\_\_

---

**THIS AMENDMENT N°1 TO THE DEVELOPMENT AND MANUFACTURING AGREEMENT** (this “**Amendment**”) is effective as of January 7<sup>th</sup>, 2011 (“**Effective Date**”).

**BY AND BETWEEN**

**ON THE ONE HAND**

(1) **INSTITUT PASTEUR** a non profit private foundation organized under the laws of France with offices at 25-28 rue du Docteur Roux, 75 724 Paris Cedex 15, France, VAT FR 65 775 684 897 (“**Institut Pasteur**”), acting herein in the name and behalf of the Consortium (“**Consortium**” and each designated individually as “**Consortium Member**”) which has been organized under an agreement by and between the following members:

**L’INSTITUT NATIONAL DE LA SANTE ET DE LA RECHERCHE MEDICALE**, Etablissement Public á caractère Scientifique et Technologique, organized under the laws of France, having its principal offices at 101 rue de Tolbiac, 75013 Paris, (“**INSERM**”),

**INSERM TRANSFERT**, Société Anonyme, organized under the laws of France, registered under RCS Paris n° 434 033 619 having its principal offices at 7, rue Watt - 75013 Paris, (“**INSERM-TRANSFERT**”),

**L’ECOLE NATIONALE VETERINAIRE ET DE L’AGROALIMENTAIRE ET DE L’ALIMENTATION DE NANTES ATLANTIQUE**, centre d’expérimentation sur l’animal en thérapie génique et cellulaire, organized under the laws of France, having its principal offices at Atlanpole - La Chantrerie - 44 307 NANTES, (“**ONIRIS**”),

**INSTITUT PASTEUR** a non profit private foundation organized under the laws of France with offices at 25-28 rue du Docteur Roux, 75 724 Paris Cedex 15, France, VAT FR 65 775 684 897 (“**INSTITUT PASTEUR**”),

**L’ASSOCIATION FRANCAISE CONTRE LES MYOPATHIES**, L’Association Francaise contre les Myopathies, an association governed by the law of July 1, 1907, *reconnue d’utilite publique de droit prive*, organized and existing under the laws of France, having its principal office at L’Institut de Myologie, 47-83 boulevard de L’Hopital, 75651 Paris Cedex 13, (**AFM**)

(“the Consortium represented herein by “**Institut Pasteur**”)]

And

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**ON THE OTHER HAND**

(2) **AMSTERDAM MOLECULAR THERAPEUTICS (AMT) BV** a company incorporated under the laws of the Netherlands, with offices at P.O.Box 22506 - 1100 DA Amsterdam, The Netherlands, (“**AMT**”).

(each, a “**Party**” and together the “**Parties**”)

**BACKGROUND:**

- (A) The Parties have signed a Development and Manufacturing Agreement dated January 7<sup>th</sup>, 2011 (hereinafter the “**Agreement**”).
- (B) The Parties have identified some points to be clarified in the Agreement.

**IT IS NOW AGREED AS FOLLOWS:**

**1      Modifications**

- 1.1. The provisions of the Article 4.1 of the Agreement is cancelled and replaced by the following :

“4.1.Quality Agreement. The Quality Agreement shall be agreed and executed within [\*\*] months following the Effective Date and shall specify certain testing, storage, release, cGMP, regulatory and other quality assurance requirements relating to manufacture and shipment of Product by AMT under this Agreement. AMT shall comply with the Quality Agreement at all times in carrying out its obligations under this Agreement. “

- 1.2. The Exhibit A of the Agreement is cancelled and replaced by the Exhibit A of this Amendment.

**2      Miscellaneous**

- 2.1. All the other provisions of the Agreement remain unchanged and fully applicable between the Parties.
- 2.2. This Amendment is effective retroactively from January 7<sup>th</sup>, 2011.
- 2.3. This Amendment makes integral part of the Agreement.

**IN WITNESS WHEREOF**, the Parties have caused this Agreement to be executed as of the Effective Date.

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By: /s/ Christophe Mauriet  
 Name: Christophe Mauriet  
 Title: Senior Executive Vice-President  
 Date: 04 ADUT 2011

## AMSTERDAM MOLECULAR THERAPEUTICS (AMT) BV

By: /s/ J. Preusling (M. Mare)  
 Name: J. Preusling M Mareli  
 Title: Dir. PMI OPS Dir. HR  
 Date: 12 Aug 11 12 Aug 11

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### Exhibit A Project Plan

Sanfilippo B or mucopolysaccharidosis type IIIB (MPSIIIB) is a lysosomal storage disease for which currently no treatment is available. The rationale for the present approach is the observation that the phenotype of MPSs can be reversed by enzyme replacement therapy. In case of Sanfilippo B the disease is caused by an autosomal recessive genetic defect of the lysosomal enzyme  $\alpha$ -N-acetylglucosaminidase (NaGlu). It was shown recently in different preclinical models that gene delivery of NaGlu has the potential to cure Sanfilippo B. The goal of the present project is to produce GMP grade AAV-NaGlu vector in sufficient amounts for clinical trials. The efficacy and safety of this vector will be tested in different animal models (for efficacy by measuring enzyme activity in MPSIIIB mice, for safety by performing toxicology and biodistribution studies in normal rats and dogs) before it will be applied in a phase I clinical trial.

#### Responsibilities

The table below outlines the main tasks and the parties responsibility for the delivery of the task.

	Task	AMT responsibility	Institut Pasteur responsibility
1	Process Development	[**]	[**]
2	Assay Development	[**]	[**]
3	<i>In vivo</i> tests in mice	[**]	[**]
	<i>Tox in rats and dogs</i>		[**]
4	MSV/WSV for cGMP batches	[**]	[**]
5	cGMP batch production	[**]	[**]

#### Assay to be developed

AAV5-specific assays will be developed by AMT; these will include:

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In addition, AMT will develop a transgene specific Q-PCR. A basic protocol has been already established at AMT, however, this process needs to be qualified prior to the initiation of the clinical phase I study.

A second Q-PCR will be established in collaboration with a service provider. This Q-PCR method will be used by Institut Pasteur in biodistribution studies in rats.

#### Develop production platform

AMT will develop a robust upstream and downstream process suitable for the production of GMP material. The progress of the development process will be monitored by Q-PCR (and in vivo studies in mice). The in vivo studies will measure the transduction efficiency of AAV5-NaGlu,

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and will be used as potency assay. Institut Pasteur will be responsible for the in vivo potency assays that will accompany the process development work. MPSIIIB mice will be used for the study. The mice will be diagnosed at birth by PCR, they will be used for the study when aged between week [\*\*]. The material from the [\*\*] development batches will be used to perform these studies. The NaGlu catalytic activity resulting from gene transfer by the AAV5-NaGlu vector will be measured in brain extracts approximately [\*\*] weeks post injection.

AMT has planned to deliver a maximum of [\*\*] development batches during this phase. The production of the [\*\*] development batch will be done adapting the AAV5-NaGlu production process previously developed at AMT.

The current AAV5 process is based on AMT's proprietary baculovirus / insect cell platform which will be used to produce the AAV5-NaGlu vector. The vector will be purified by DNA-ase incubation followed by a chemical lysis step after which cell debris is removed by depth filtration. The clarified harvest is incubated overnight as a viral inactivation step. The inactivated material is purified by affinity chromatography and ion-exchange chromatography after which buffer exchange is performed using ultrafiltration/diafiltration (UF/DF). The intended formulation buffer consists of phosphate buffered saline (PBS) and [\*\*]. After UF/DF the product is frozen at [\*\*] and samples are analyzed according to a pre-determined list of analyses and specification. The result of the Q-PCR analysis

Following the delivery of the batch, Institut Pasteur will test the batch in vivo in a mouse model (in vivo potency study). If needed, further process optimization will be employed for the production of the [\*\*] development batch, which will be again tested as above. Final process optimization work will result in the production of the [\*\*] development batch, which will be again tested as above. The process used for the most successful in vivo study (highest NaGlu activity per mg of protein in extracts prepared from the entire injected brain hemisphere) will be employed for the Tox studies. The same process will be taken into a GMP environment for the production of clinical material. Purchasing new equipment for optimization of the manufacturing process is not included in the scope of this project.

## 1. Development batches

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## 2. Analytical development

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A typical bill of testing for the finished product is listed in the Table below. Some process related impurities such as residual Benzonase, Triton, infectious baculovirus are tested on the active substance level and safety assays such as adventitious virus agents are tested during the manufacturing process on the bulk harvest prior to addition of Triton. AMT and Institut Pasteur have to agree on the number of (critical) in process controls for which AMT will make a proposal for discussion.

Test parameter	Tentative Acceptance Criteria
General tests and tests for contamination	
[**]	[**]
[**]	[**]
[**]	[**]
[**]	[**]
[**]	[**]
[**]	[**]
[**]	[**]
	[**]
[**]	[**]
	[**]
[**]	[**]
[**]	[**]
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[**]	[**]
[**]	[**]
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[**]	[**]
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[**]	[**]

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[**]	[**]
[**]	[**]
	[**]
[**]	[**]
[**]	[**]

Please note that the above table provides estimate acceptance criteria. Exact acceptance criteria will need to be discussed and agreed in the project team but with no substantial changes.

### 3. MSV / WSV production

AMT will produce a GMP compliant Master Seed Virus and Working Seed Virus Bank of at least [\*\*] vials of the baculovirus containing the NaGlu gene cassette. The MSV and WSV will be tested according to EP requirements and similar to previous virus banks which were qualified by AMT. The GMP compliant AAV5 and Rep containing MSV and WSV baculoviruses are already available for the production of the NaGlu vector.

### 4. GMP batch

Because of the small amounts of product that are expected for a clinical phase I study and because it is expected that the AAV5-NaGlu product is stable for a long period of time at conditions of [\*\*] or lower, [\*\*] cGMP batch will be produced for supply the clinical material. In addition to the batch production, the appropriate testing will be done as required. AMT and Institut Pasteur will have to agree on the final bill of testing for the clinical drug product. AMT will release the product to Institut Pasteur based on meeting the specifications on the bill of testing. Acceptance of the batch by Institut Pasteur is done based on AMT release statement.

### 5. Stability study

AMT will conduct a stability study on the material used in the Tox study to prove that the product was still stable upon the start of the study, and to determine a preliminary shelf life. The clinical batch will also be put on stability for a period of a maximum [\*\*] months and will be periodically tested for stability. Based on the outcome, the finished product can be given a shelf life of [\*\*] months.

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### 6. Reference standard

AMT will use some material of the GMP batch for the initial reference standard and will qualify the reference standard based on a protocol which will have to be agreed between Institut Pasteur and AMT.

### 7. Tox and safety studies

Not included in AMT's project scope. Tox study will be performed under responsibility of Institut Pasteur. AMT can contribute advice upon request based on its experience in conducting gene therapy Tox and safety studies.

### 8. Phase I/II clinical study

Not included in AMT's project scope. Clinical study and all its related activities (analysis of clinical samples etc.) will be performed under responsibility of Institut Pasteur. AMT can contribute advice upon request based on its experience in conducting gene therapy clinical trial, for up to [\*\*] hours without charge.

### 9. Viral clearance / inactivation study

AMT will produce [\*\*] using an identical recipe to the cGMP clinical batch to generate materials for an adventitious viral agents removal study and for a baculovirus removal study. The [\*\*] study will be outsourced to a qualified contract laboratory. In the [\*\*] study one enveloped and one unenveloped model virus will be tested for inactivation and clearance by the process (singular experiment). AMT will report the first study and will make the reports available for submission of the IMPD or equivalent application for the clinical study by Institut Pasteur.

## Regulatory Affairs Strategy

Contacts to AFSSAPS have already been established by Institut Pasteur. Institut Pasteur will be responsible for the interaction with AFSSAPS. Institut Pasteur will keep AMT informed about any advice received that relates to manufacturing.

Institut Pasteur is responsible for the orphan drug application. AMT will contribute information related to product development and manufacturing as part of this project, if any.

## QP Release

AMT, as contract manufacturer, will release the manufacturing data as well as the quality control data generated by AMT or AMT's vendors. The QP of AMT will provide Institut Pasteur with a certificate stating that production has taken place according to GMP and test results comply with the specification.

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Project Plan - Delivery Dates

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Milestones based on start of practical activities on [\*\*]:

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Stability study: proposal for criteria for [\*\*] month study at timepoints [\*\*]

Stability acceptance criteria

Test parameter	Acceptance Criteria	Method
General tests		
[**]	[**]	[**]
[**]	[**]	[**]
[**]	[**]	[**]
[**]		
[**]	[**]	[**]
[**]	[**]	[**]
[**]	[**]	[**]
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[**]	[**]	[**]
[**]	[**]	[**]

[**]		
[**]	[**]	[**]
[**]	[**]	[**]
[**]		
[**]	[**]	[**]

Development costs

The estimate costs for development and production of the clinical batch(es) and licensing costs are in euros and are:

	Batch [**]	[**]	Batch [**]
Development (including Quality Agreement):			
Process development	[**]		
Assay development	[**]		
Virus removal study	[**]		
Stability study	[**]	[**]	[**]

Preliminary ref. standard qualification [**] GMP batch	[**]		
Release assays [**]		[**]	
<b>Manufacture:</b>			
[**] GMP batch	[**]		[**]
IMPD / IB	[**]		
Qualification of analytics	[**]		[**]
Overheads on FTE	[**]		
QP costs		[**]	
<b>Total Cost estimate</b>	[**]	[**]	[**]

Discount

AMT recognise the substantial time committed to this project to date by all parties. In the interests of trying to support this project going forward, AT is prepared to offer [\*\*] against the costs of each of Batch [\*\*] and Batch [\*\*] above.

Payment Plan

Signature fee		[**]
[**]		[**]
[**]		[**]
[**]		[**]
[**]		[**]
[**]		[**]

Additional Services

Additional storage of Batches, or any other Institut Pasteur owned property (Raw Materials, Product, etc) beyond the first [\*\*] month period : €[\*\*] per month per [\*\*] volume (or part thereof)

Provision of documents or other work product (other than the MPR, the Manufacturing Documentation and copies of Batch Disposition Documentation and any other documents or work product expressly required to be delivered under this Agreement) that do not exist as of the date of

request or other substantive requests for assistance in compiling any Regulatory Filing : €[\*\*]/hour for AMT staff together with reimbursement of third party expenses as invoiced to AMT.

Additional manufacturing audits : €[\*\*]/hour for AMT staff together with reimbursement of third party expenses as invoiced to AMT.

Confidential Materials omitted and filed separately with the Securities and Exchange Commission. Double asterisks denote omissions.

## LICENSE AGREEMENT

### Preamble

This License Agreement (this “**Agreement**”) effective as of November 30, 2010 (the “**Effective Date**”), is made by and between Amsterdam Molecular Therapeutics (AMT) B.V., a closed limited liability company with registered offices at Meibergdreef 61, 1100 DA Amsterdam, the Netherlands (“**AMT**”), and Amgen Inc., a Delaware corporation whose address is One Amgen Center Drive, Thousand Oaks, CA 91320-1799, USA (“**Amgen**”). AMT and Amgen are sometimes referred to herein individually as a “**Party**” and collectively as the “**Parties**”.

### Recitals

**WHEREAS**, AMT is a company engaged in the research, development, manufacturing and commercialization of gene therapy products, including research and development of therapeutics for the treatment of neurological diseases and other diseases; and

**WHEREAS**, Amgen is a company engaged in the research, development, manufacturing and commercialization of pharmaceutical and biotechnology products, including research and development of therapeutics for the treatment of neurological diseases and other diseases; and

**WHEREAS**, Amgen has developed glial-cell derived neurotrophic factor (“**GDNF**”) for the treatment of neurological diseases and has certain rights to Licensed Patent Rights and Licensed Know-How (each as defined below), but Amgen is not currently engaged in the Exploitation (as defined below) of any gene therapy treatment with respect to GDNF; and

**WHEREAS**, Amgen and AMT previously entered into that certain License and Collaboration Agreement dated as of September 11, 2008 (the “**Original Agreement**”) pursuant to which the Parties agreed to participate in a collaboration relating to the development and production of a recombinant adeno-associated virus (“**AAV**”) that delivers GDNF; and

**WHEREAS**, the Parties wish to terminate the Original Agreement and by this Agreement to provide for a license by Amgen to AMT under the Licensed Patent Rights and the Licensed Know-How for the Exploitation of GDNF Products (as defined below).

**NOW, THEREFORE**, the Parties in consideration of the mutual representations, warranties and covenants contained herein and for other good and valuable consideration, hereby agree as follows:

## **ARTICLE 1. DEFINITIONS**

The following terms shall have the meanings set forth below:

**SECTION 1.1** “**AAV**” has the meaning set forth in the Recitals.

Amgen Contract No.: 2010549620

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**SECTION 1.2** “**Affiliate**” means any Person which controls, is controlled by or is under common control with a Party. For purposes of this Section 1.2 (“**Affiliate**”), “**control**” shall mean (i) in the case of corporate entities, direct or indirect ownership of fifty percent (50%) or more of the stock or shares entitled to vote for the election of directors and (ii) in the case of non-corporate entities, direct or indirect ownership of fifty percent (50%) or more of the equity or income interest therein. Notwithstanding the preceding provisions, with respect to an Affiliate of a Party to this Agreement, once such entity ceases to be an Affiliate of such Party, then, without any further action, such entity shall cease to have any rights, including license and sublicense rights, under this Agreement by reason of being an Affiliate.

**SECTION 1.3** “**Agreement**” has the meaning set forth in the Preamble.

**SECTION 1.4** “**Amgen**” has the meaning set forth in the Preamble.

**SECTION 1.5** “**Amgen Indemnified Parties**” has the meaning set forth in Section 9.9.2 (By AMT).

**SECTION 1.6** “**AMT**” has the meaning set forth in the Preamble.

**SECTION 1.7** “**AMT Improvements**” means all Know-How and Materials comprising or relating to any results generated solely by or on behalf of AMT in its Exploitation of GDNF Products up to the date of any termination of this Agreement comprising (i) construction of the baculoviruses required for manufacturing of a GDNF Product in insect cells and any other process development or optimization of AMT’s baculovirus-based vector production system in insect suspension culture (upstream) or affinity chromatography-based purification system (downstream); or (ii) improvements and characterization of AMT’s AAV5 platform, particularly for CNS applications; in each case including but not limited to all improvements, modifications and developments generated solely by or on behalf of AMT in its Exploitation of GDNF Products up to the date of any termination of this Agreement to the AMT Licensed Know-How (including AMT Manufacturing Technology) and Materials comprising AMT Licensed Know-How as of the Original Agreement Effective Date relating to the subject matter described in clauses (i) and (ii) above.

**SECTION 1.8** “**AMT Indemnified Parties**” has the meaning set forth in Section 9.9.1 (By Amgen).

**SECTION 1.9** “**AMT Licensed Know-How**” means all Know-How including AMT Manufacturing Technology and Materials that is Controlled by AMT and/or its Affiliates during the term of this Agreement including AMT Improvements.



**SECTION 1.10** “**AMT Licensed Patent Rights**” means (i) (a) the patents and patent applications listed on Schedule 1.10 and any patent applications from which any such patents or patent applications claim priority and any patent applications filed by AMT claiming or otherwise in relation to AMT Improvements; (b) all patent applications filed in any jurisdiction corresponding to or claiming priority directly or indirectly from any patent and/or patent

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application referred to in the foregoing clause (a); (c) all divisionals, continuations and continuations-in-part of the patent applications referred to in the foregoing clauses (a) and (b); (d) all patents issuing from the patent applications referred to in the foregoing clauses (a), (b) and (c); and (e) all reissues, re-filings, re-examination certificates, registrations, confirmations, extensions, substitutions, renewals, foreign counterparts, and supplementary protection certificates of the patents and/or patent applications referred to in the foregoing clauses (a), (b), (c) and (d), and (ii) any and all patents and/or patent applications Controlled during the term of this Agreement by AMT and/or its Affiliates that would (absent the licenses granted herein) be infringed by the Exploitation of a GDNF Product or the infringement of which would (absent the licenses granted herein) be induced or contributed to by the Exploitation of a GDNF Product. For purposes of determining whether a patent application falls within this definition, a patent application shall be considered “infringed” if its pending claims would be infringed if issued as then currently set forth in the patent application.

**SECTION 1.11** “**AMT Manufacturing Technology**” means any of the following that are reasonably necessary for manufacturing of GDNF Products: (i) AAVs, virions, plasmids, host cells, packaging cells, or components of any of the foregoing, reagents, assays, processes, and protocols; (ii) data, including in vivo and in vitro data produced by or on behalf of AMT or its Affiliates; and (iii) any other documentation (including any CMC sections of regulatory filings) or materials in each case (i), (ii) and (iii) Controlled by AMT or its Affiliates.

**SECTION 1.12** “**Bankruptcy Code**” has the meaning set forth in Section 9.21 (License to Intellectual Property).

**SECTION 1.13** “**Bundle**” means a GDNF Product (a) sold together with another pharmaceutical product for a single price including fixed combinations or (b) sold as part of a delivery agent (such as a viral vector) which contains two or more different therapeutic genes or therapeutic fragments of genes and not all of the therapeutic genes or fragments of genes encode GDNF or a fragment of GDNF that has Functional Activity.

**SECTION 1.14** “**Business Day**” means a calendar day that is neither (a) a public holiday in New York, New York, (b) a recognized Federal holiday in the U.S. (b) a government-recognized holiday in the Netherlands, nor (d) a formal company-specific holiday of a Party, including Amgen’s voluntary holiday shut downs.

**SECTION 1.15** “**Calendar Quarter**” means each respective period of 3 consecutive months ending on March 31, June 30, September 30 and December 31 of each Calendar Year.

**SECTION 1.16** “**Calendar Year**” means each respective period of 12 months commencing on January 1 and ending on December 31.

**SECTION 1.17** “**CMO**” has the meaning set forth in Section 3.1 (Manufacturing).

**SECTION 1.18** “**COGS**” means all direct costs actually incurred by AMT and/or its Affiliates (and for the purposes of Section 4.2(iii), and/or its Sublicensees) in (i) the manufacture (or

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procurement from Third Parties) of GDNF Products, including purchase of raw materials used to manufacture GDNF Products, payments to Third Party subcontractors for the manufacture of GDNF Products and (ii) the provision of services using GDNF Products, but in each case excluding Third Party License Payments; **provided, however**, that (A) “COGS” shall not include any amounts attributable to (i) idle or underutilized facilities, (ii) general corporate overhead, or (iii) equity-based compensation provided to any employee, officer or director of AMT and/or its Affiliate (and for the purposes of Section 4.2(iii), and/or its Sublicensees), and (B) in determining “COGS,” amounts related to or attributable to employees of AMT and/or its Affiliates (and for the purposes of Section 4.2(iii), and/or its Sublicensees) shall only be included to the extent they are Employment Costs allocable to personnel directly involved in the manufacture of GDNF Products such as equipment operators, line mechanics, set up mechanics and material handlers to supply the line, and amounts allocable to time during which such Product-Related Personnel conduct other activities (including without limitation manufacture of other products), as well as Employee Costs for any other personnel, shall be excluded.

**SECTION 1.19** “**Collaboration Option**” has the meaning set forth in Section 2.6 (Collaboration Option).

**SECTION 1.20** “**Collaboration Territory**” means the U.S., Mexico and Canada.

**SECTION 1.21** “**Completion of the first Phase 2 Clinical Trial**” means completion and approval of the Clinical Study Report as defined in ICH E6 or its equivalent in any jurisdiction where such Phase 2 Clinical Trial is carried out.

**SECTION 1.22** “**Confidential Information**” means all proprietary information (whether or not patentable) communicated to either Party by or on the behalf of the other Party and indicated in writing as “Confidential” (or, if disclosed orally or visually, indicated in a written summary as confidential within [\*\*] days of the initial disclosure thereof), including: formulations, techniques, methodology, equipment, instrumentation, data, reports, know-how, sources of supply, patent positioning, business plans and projections, Sublicense terms, Payment Reports, and strategy and other information about prosecution of the Licensed Patent Rights, including the content of any communications under Section 7.1 (Patent Prosecution).

**SECTION 1.23** “**Control**” means with respect to any Know-How (including any Licensed Know-How), Material or intellectual property rights (including Licensed Patent Rights) to which either Party has rights as of the Original Agreement Effective Date or obtains rights after the Original Agreement Effective Date but before termination of this Agreement, possession by a Party or its Affiliate of the ability (whether by ownership, license or otherwise) to grant access, a license or a sublicense to such Know-How, Material or intellectual property right as provided for in this Agreement (i) without violating the terms of any agreement with any Third Party in existence as of the Original Agreement Effective Date and (ii) without requiring any further payment (whether or not then due and payable) under any agreement with any Third Party (unless Amgen has, in accordance with Section 8.5.1.1 (Grant of License), elected to pay AMT any

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amounts owed to such Third Party under the Third Party Agreements by reason of Amgen's Exploitation of GDNF Products).

**SECTION 1.24** "**Cover**" means that the Exploitation of a GDNF Product within the Licensed Field would infringe or induce or contribute to the infringement of a Patent Right absent a license thereof.

**SECTION 1.25** "**Data Package**" has the meaning set forth in Section 2.6 (Collaboration Option).

**SECTION 1.26** "**Defending Party**" has the meaning set forth in Section 7.2 (Defense of Third Party Infringement Claims).

**SECTION 1.27** "**Diligence Notice**" has the meaning set forth in Section 5.5 (Diligence Schedule).

**SECTION 1.28** "**Divest**" means, with respect to any Subsequent Distracting Program, the sale, exclusive license or other transfer of all of the right, title and interest in and to such Subsequent Distracting Program, as applicable, including technology, know-how, patent(s), trademark(s), and other assets solely relating thereto, (except to the extent that such other assets have uses other than for the Subsequent Distracting Program) to an independent Third Party by AMT or its Affiliates without the retention or reservation of any rights or interest, including not exercising nor having the ability to materially participate in or advise with respect to the research, development, manufacture or commercialization of any product in the Subsequent Distracting Program (other than solely an economic interest, the right to enforce customary terms and conditions contained in the relevant agreements effectuating such Divestiture (such as obligations to use diligent efforts to develop and/or commercialize the Subsequent Distracting Program) and customary reversionary rights in the event of certain terminations). For avoidance of doubt, co-development and co-marketing arrangements are not considered transactions that Divest a Subsequent Distracting Program.

**SECTION 1.29** "**Effective Date**" has the meaning set forth in the Preamble.

**SECTION 1.30** "**EMA**" means the European Medicines Agency and any successor agency.

**SECTION 1.31** "**Employment Costs**" means actual costs incurred by AMT and/or its Affiliates with respect to any employee, but excluding any allocable overhead.

**SECTION 1.32** "**Enforcement Action**" has the meaning set forth in Section 7.3 (Enforcement).

**SECTION 1.33** "**Enforcing Party**" has the meaning set forth in Section 7.3 (Enforcement).

**SECTION 1.34** "**Exploit**" means to research, have researched, develop, have developed, make, have made, use, have used, offer for sale, have offered for sale, sell, have sold, import, have

imported, export have exported or otherwise exploit, or transfer possession of or title in, a product. Cognates of the word "**Exploit**" shall have correlative meanings.

**SECTION 1.35** "**FDA**" means the United States Food and Drug Administration and any successor agency.

**SECTION 1.36** "**Foreign Currency Amount**" has the meaning set forth in Section 6.3 (Exchange Rate; Manner and Place of Payment).

**SECTION 1.37** "**Functional Activity**" means neurotrophic activity with respect to dopaminergic neurons with an ability to (i) signal through the GDNF receptor complex and (ii) (a) increase tyrosine hydroxylase immunoreactivity or (b) protect against neurotoxin-mediated cell death.

**SECTION 1.38** "**IFRS**" means International Financial Reporting Standards, consistently applied, as used by AMT to record the relevant transaction.

**SECTION 1.39** "**Infringing Product**" has the meaning set forth in Section 7.3 (Enforcement).

**SECTION 1.40** "**GDNF**" has the meaning set forth in the Recitals.

**SECTION 1.41** "**GDNF Product**" means any Gene Therapy product capable of delivering GDNF or the gene encoding GDNF, or in either case any fragment of GDNF that has Functional Activity.

**SECTION 1.42** "**Gene Therapy**" means therapy conducted by means of delivery (by any means, including ex vivo, in vivo or otherwise) of one or more nucleic acid sequences (including, DNA, RNA or any hybrids thereof and combinations with other chemical entities or macromolecules, whether coding or noncoding).

**SECTION 1.43** "**Governmental Authority**" means any government or supranational administrative agency, commission or other governmental or supranational authority, body or instrumentality, or any federal, state, local, domestic or foreign governmental or supranational regulatory body.

**SECTION 1.44** "**IND**" means an Investigational New Drug Application (including any amendments thereto) submitted to the **FDA or any comparable** filings with a Governmental Authority in any country outside the U.S. for a drug or other therapy, before the commencement of clinical trials involving a GDNF Product.

**SECTION 1.45** "**Know-How**" means data, inventions, know-how, trade secrets, methods, knowledge and information (including chemical manufacturing data, toxicological data, pharmacological data, preclinical data, assays, platforms, formulations, specifications, and quality control testing data), in each case reasonably necessary for the Exploitation of GDNF Products.

**SECTION 1.46** “**Launch**” means the date of the first commercial sale by AMT, its Affiliate or its Sublicensee of a GDNF Product.

**SECTION 1.47** “**Law**” means, individually and collectively, any and all laws, ordinances, rules, directives, administrative circulars and regulations of any kind whatsoever of any Governmental Authority within the applicable jurisdiction together with the rules and regulations of any securities exchange.

**SECTION 1.48** “**Licensed Field**” means all uses in Gene Therapy.

**SECTION 1.49** “**Licensed Know-How**” means any Know-How that (i) is Controlled by Amgen and/or its Affiliates and (ii) was provided by Amgen to AMT under the Original Agreement (but only to the extent expressly designated in writing by Amgen as “Amgen Licensed Know-How”). For the avoidance of doubt, the cell lines 32D and NGR-38 and the protocols outlining the methodology for the purposes of assessing the biological activity of the GDNF construct are Licensed Know-How.

**SECTION 1.50** “**Licensed Patent Rights**” means (i) the patents and patent applications listed on Schedule 1.50 and any patent applications from which any such patents or patent applications claim priority; (ii) all patent applications filed in any jurisdiction corresponding to or claiming priority directly or indirectly from any patent and/or patent application referred to in the foregoing clause (i); (iii) all divisionals, continuations and continuations-in-part of the patent applications referred to in the foregoing clauses (i) and (ii); (iv) all patents issuing from the patent applications referred to in the foregoing clauses (i), (ii) and (iii); (v) all reissues, re-filings, re-examination certificates, registrations, confirmations, extensions, substitutions, renewals, foreign counterparts, and supplementary protection certificates of the patents and/or patent applications referred to in the foregoing clauses (i), (ii), (iii) and (iv); (vi) any other Patent Rights Controlled by Amgen and/or its Affiliates as of the Effective Date necessary for the Exploitation of a GDNF Product (but only to the extent such Patent Rights Cover any GDNF Product as developed by AMT as of the Effective Date); and (vii) any other Patent Rights Controlled by Amgen and/or its Affiliates during the Payment Term that have been conceived, generated or reduced to practice in the course of any research conducted in accordance with Section 2.2 and that are necessary or useful for the Exploitation of a GDNF Product.

**SECTION 1.51** “**Losses**” has the meaning set forth in Section 9.9.1 (By Amgen).

**SECTION 1.52** “**Materials**” means biological materials or chemical compounds including GDNF AAVs, virions, plasmids, host cells, packaging cells, or components of any of the foregoing, assays, screens, data, protocols, knock-out mice and other animal models, cell lines, cells, nucleic acids, receptors and reagents.

**SECTION 1.53** “**NDA**” means a New Drug Application, Biological License Application or other applicable regulatory filing submitted to the FDA, the EMA or any equivalent entity outside the U.S. and the European Union for a drug or other therapy, which if granted, would be sufficient to permit under applicable Law the promotion and/or sale of such drug or other therapy.

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**SECTION 1.54** “**Net Revenues**” means Net Sales and Net Service Revenues. Notwithstanding the foregoing, in the event that Amgen exercises the Collaboration Option, Net Revenues shall exclude any Net Revenues achieved by Amgen, its Affiliates and/or their respective sublicensees in the Collaboration Territory.

**SECTION 1.55** “**Net Sales**” means the gross invoiced sales prices charged for GDNF Products sold by or for AMT and/or its Affiliates (and for the purposes of Section 4.2(iii), and its Sublicensees) in arms length transactions to Third Parties (but not including sales relating to transactions between AMT, its Affiliates (and for the purposes of Section 4.2(iii), its Sublicensees), and/or their respective agents, unless for end use consumption), less the total of the following charges or expenses as determined in accordance with IFRS: (i) trade, cash, prompt payment and/or quantity discounts including promotional or service discounts; (ii) returns, allowances, rebates, chargebacks, or payments to government agencies; (iii) retroactive price reductions applicable to sales of such product; (iv) fees paid to distributors, selling agents (excluding any sales representatives of AMT or any of its Affiliates (or for the purposes of Section 4.2(iii), its Sublicensees)), group purchasing organizations and managed care entities or similar types of organizations; (v) credits or allowances for product replacement, whether cash or trade; (vi) non-recoverable sales taxes, excise taxes, tariffs and duties (excluding taxes when assessed on income derived from sales); and (vii) bad debt, freight or other transportation charges, insurance charges, additional special packaging, and other governmental charges. Notwithstanding the foregoing, the Parties intend that Net Sales exclude amounts invoiced for or revenues attributable to services ancillary to the delivery or use of GDNF Products including surgical procedures, hospital stays or the like, as well as amounts invoiced for devices sold in connection with any GDNF Product. If AMT (or its Affiliates (or for the purposes of Section 4.2(iii), its Sublicensees)) sells a GDNF Product to a Third Party to whom it also provides other products or services (including any device used or sold in connection with such GDNF Product), AMT (and its Affiliates (and for the purposes of Section 4.2(iii), its Sublicensees)) shall not price, discount or otherwise offer (including bundling) the GDNF Product in any way that benefits such other products or services at the expense of such GDNF Product or otherwise disadvantages Amgen with respect to royalties that would be paid to Amgen hereunder. In all events, AMT (and its Affiliates (and for the purposes of Section 4.2(iii), its Sublicensees)) shall price and offer GDNF Products sold by it hereunder in a manner consistent with standard practices in the pharmaceutical industry and applicable Law.

Any disposal of GDNF Products at no charge for, or use of GDNF Products at no charge in clinical or pre-clinical trials, and GDNF Products given as free samples, or distributed at no charge to patients unable to purchase GDNF Product shall not be included in Net Sales.

Upon any sale or other disposal of any GDNF Product for any consideration other than an exclusively monetary consideration on bona fide arm’s length terms, then for purposes of calculating the Net Sales under this Agreement, such GDNF Product shall be deemed to be sold at the average sales price for such GDNF Product having the same dosage form and strength during the applicable reporting period in the country where such sale or other disposal occurred when such GDNF Product is sold alone and not with other products, and if such GDNF Product is not

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sold alone in such country during the applicable reporting period, then such GDNF Product shall be deemed to be sold at the average sales price during the applicable reporting period generally achieved for such GDNF Product having the same dosage form and strength in the rest of the world.

Where a GDNF Product is sold in a Bundle, then for the purposes of calculating the Net Sales under this Agreement, such GDNF Product shall be deemed to be sold for an amount equal to  $(X/(X+Y)) * Z$ , where: X is the average sales price during the applicable reporting period for such GDNF Product being sold alone (in the same dosage form) (or, should more than one GDNF Product be included in a Bundle with a product other than a GDNF Product, the sum of such average sales prices for the included GDNF Products) in the particular country of sale; Y is the sum of the average sales price during the applicable reporting period in the

particular country of sale, when sold alone, of each pharmaceutical (other than the included GDNF Product(s)) included in the Bundle (in the same dosage form); and Z equals the Net Sales of such Bundle. In the event that a GDNF Product or one or more of the other pharmaceuticals in the Bundle are not sold separately (in the same dosage form), the Parties will discuss in good faith to determine an equitable fair market price to apply to such GDNF Product or other pharmaceutical in the Bundle.

**SECTION 1.56** “*Net Service Revenues*” shall mean the revenues from any use of a GDNF Product in the provision of a service performed by or for AMT and its Affiliates and Sublicensees, to the extent not taken into account as Net Sales, less the total of the following charges or expenses as determined in accordance with IFRS, in each case as they relate to the provision of such service using a GDNF Product during such time period: (i) trade, cash, prompt payment and/or quantity discounts including promotional or service discounts; (ii) allowances, rebates, chargebacks, or payments to government agencies; and (iii) fees paid to selling agents (excluding any representatives of AMT or any of its Affiliates or Sublicensees), group purchasing organizations and managed care entities or similar types of organizations. Notwithstanding the foregoing, the Parties intend that Net Service Revenues exclude amounts invoiced for or revenues attributable to services ancillary to the delivery or use of GDNF Products including surgical procedures, hospital stays or the like, as well as amounts invoiced for devices sold in connection with any GDNF Product. If AMT (or its Affiliates or Sublicensees) uses a GDNF Product in connection with the provision of a service to a Third Party to whom it also provides other products or services (including any device used or sold in connection with such GDNF Product), AMT (and its Affiliates and Sublicensees) shall not price, discount or otherwise offer (including bundling) the GDNF Product in any way that benefits such other products or services at the expense of such GDNF Product or otherwise disadvantages Amgen with respect to royalties that would be paid to Amgen hereunder. In all events, AMT (and its Affiliates and Sublicensees) shall price and offer services using GDNF Products hereunder in a manner consistent with standard practices in the pharmaceutical industry and applicable Law.

Where AMT (or its Affiliates or Sublicensees) uses a GDNF Product in a Bundle in connection with the provision of a service to a Third Party, then for the purposes of calculating the Net Service Revenues under this Agreement, such service shall be deemed to be provided for an amount equal to  $(X/(X+Y)) * Z$ , where: X is the average sales price during the applicable reporting period for the

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services in relation to such GDNF Product being provided alone (or, should more than one GDNF Product be included in a Bundle with a pharmaceutical product other than a GDNF Product, the sum of such average sales prices for the included GDNF Product) in the particular country of the provision of the service; Y is the sum of the average sales price during the applicable reporting period in the particular country of the provision of the service, when sold alone, of each pharmaceutical product (other than the included GDNF Product(s)) included in the Bundle; and Z equals the Net Service Revenues of such Bundle. In the event that a service in connection with a GDNF Product or one or more of the other pharmaceutical products in the Bundle are not sold separately, the Parties will discuss in good faith to determine an equitable fair market price to apply to such GDNF Product services or other pharmaceutical products in the Bundle. For the purposes of this definition, a GDNF Product service upon which Net Service Revenues are payable is that service directly related to the use of the GDNF Product and not any ancillary services. For example, but not by way of limitation, a GDNF Product service would be the injection of a GDNF Product into a patient but would not cover any fees payable for the patient’s hospital stay or for other procedures carried out on the patient, for example to check that they are suitable for treatment.

**SECTION 1.57** “*Original Agreement*” has the meaning set forth in the Recitals.

**SECTION 1.58** “*Original Agreement Effective Date*” means September 11, 2008.

**SECTION 1.59** “*Partnered Net Revenues*” means any Net Revenue attributable to the sale of a GDNF Product that is the subject of a profit and loss allocation between AMT and any Sublicensee. For the avoidance of doubt, Partnered Net Revenues does not include any Sublicensing Revenue, in particular royalties or milestones paid by a Sublicensee to AMT.

**SECTION 1.60** “*Party*” has the meaning set forth in the Preamble.

**SECTION 1.61** “*Patent Rights*” means any patent applications and any patents issuing from such patent applications, author certificates, inventor certificates, utility certificates, improvement patents and models, and certificates of addition and all counterparts of them throughout the world, including any divisional applications and patents, filings, renewals, continuations, continuations-in-part, patents of addition, extensions, reissues, substitutions, confirmations, registrations, revalidation and additions of or to any of them, as well as any supplementary protection certificates and equivalent protection rights in respect of any of them.

**SECTION 1.62** “*Payment Report*” has the meaning set forth in Section 6.1 (Payment; Reports).

**SECTION 1.63** “*Payment Term*” means, with respect to a Licensed Product in a given country, the period of time commencing on Launch of such Licensed Product in such country and continuing until the later of (a) expiration of the last to expire Licensed Patent Right issued in such country containing at least one Valid Claim Covering the GDNF Product and (b) the tenth (10th) anniversary of Launch of such GDNF Product in such country.

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**SECTION 1.64** “*Person*” means an individual, corporation, partnership, limited liability company, trust, business trust, association, joint stock company, joint venture, pool, syndicate, sole proprietorship, unincorporated organization, governmental authority or any other form of entity not specifically listed herein.

**SECTION 1.65** “*Phase 2 Clinical Trial*” means, with respect to the United States, any human clinical trial conducted in the specific patient population with the disease or condition of interest intended to be studied in a Phase 3 Clinical Trial for the purposes of preliminary assessment of efficacy in the indication being studied, as described under 21 C.F.R. §312.21(b) (or its successor regulation), and that, if the defined end-points are met, is sufficient to allow the initiation of a Phase 3 Clinical Trial in the indication being studied, or, with respect to a jurisdiction other than the United States, an equivalent clinical study.

**SECTION 1.66** “*Phase 3 Clinical Trial*” means, with respect to the United States, any human clinical trial, that, if the defined end-points are met, is designed and intended to be a pivotal trial to support the filing of an application to obtain Regulatory Approval with the FDA (or its successor) as required under 21 C.F.R. §312.21 (c) (or its successor regulation), or, with respect to a jurisdiction other than the United States, an equivalent clinical study.

**SECTION 1.67** “*Reasonably Diligent Efforts*” means, with respect to a particular GDNF Product and those activities hereunder with respect to such GDNF Product including development and commercialization, those efforts and resources that AMT would normally use with respect to a pharmaceutical product, which product is owned by AMT or to which AMT has similar rights with a similar market potential at a similar stage in development or product life as the GDNF

Product, taking into account all relevant factors, based on the facts and circumstances at the time, including without limitation safety and efficacy issues, other medical and clinical considerations, product labeling or anticipated labeling, product profile, present and future market potential, past performance of similar products (including both GDNF Products and AMT's own pharmaceutical products that are of similar market potential), competitive market conditions, the likely timing of the product's entry into the market, financial considerations, intellectual property considerations and the likelihood of regulatory approval.

**SECTION 1.68** **"Regulatory Approval"** means, in the applicable country, any approvals (including pricing and reimbursement approvals, where necessary to initiate marketing activities), licenses, registrations or authorizations of any national or international or local regulatory agency, department, bureau or other governmental entity, necessary and sufficient for the marketing and sale of a GDNF Product by AMT, its Affiliate or Sublicensee.

**SECTION 1.69** **"Regulatory Filings"** means all applications, filings, dossiers and the like (excluding routine adverse event expedited or periodic reporting), submitted to a Governmental Authority for the purpose of obtaining Regulatory Approval from that Governmental Authority in the Licensed Field.

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**SECTION 1.70** **"Revenue Sharing Payments"** has the meaning set forth in Section 4.2 (Revenue Sharing).

**SECTION 1.71** **"Sublicense"** means a grant of any right or license to Exploit GDNF Products under the Licensed Patent Rights and/or the Licensed Know-How and/or a grant of any right or license to Exploit GDNF Products under the AMT Licensed Patent Rights and/or AMT Licensed Know-How. Likewise, **"Sublicensee"** means the Person to whom rights are granted under a Sublicense. For the avoidance of doubt, the grant of a right or license with respect to any one or more activities identified within the definition of "Exploit" shall be a "Sublicense" for purposes of this Agreement, regardless of whether such Sublicense conveys to the applicable Sublicensee a right or license with respect to all activities within the definition of "Exploit." By way of illustration and not limitation, the granting to a Third Party of a license to sell, but not manufacture, GDNF Products shall be a "Sublicense." For the sake of clarity, unless the Parties agree otherwise, Amgen's election to exercise the Collaboration Option shall not result in Amgen being a Sublicensee for the purposes of this Agreement.

**SECTION 1.72** **"Sublicensing Revenue"** means in respect of any GDNF Products, all cash payments received, and the fair market value of all non-cash consideration (excluding the fair market value of any monies paid to AMT in subscription for new AMT equity or other instruments capable of conversion into AMT equity) received, by AMT and/or any of its Affiliates from any Third Party in consideration for a transaction, series of transactions or other arrangement in which such Third Party obtains (i) a Sublicense of the Licensed Patent Rights and/or Licensed Know-How (or any option or other right to obtain a Sublicense of the Licensed Patent Rights and/or Licensed Know-How) and/or (ii) a license of the AMT Licensed Patent Rights and/or AMT Licensed Know-How (or any option or other right to obtain a license of the AMT Licensed Patent Rights and/or AMT Licensed Know-How), including, without limitation, up-front payments, milestones, royalties, research funding (provided that with respect to research funding payments, only the amounts in excess of AMT's external costs and internal costs directly related to such research activities shall be included), and any monies paid to AMT in subscription for new AMT equity or other instruments capable of conversion into AMT equity in excess of the fair market value thereof (provided, however, that Sublicensing Revenue shall exclude Partnered Net Revenues). Notwithstanding the foregoing, in the event that Amgen exercises the Collaboration Option, Sublicensing Revenues shall exclude any payments made by Amgen, its Affiliates and/or their respective sublicensees to AMT in respect of the Collaboration Territory.

**SECTION 1.73** **"Subsequent Distracting Product"** means a Gene Therapy product that delivers GDNF or the gene encoding GDNF, or in either case any fragment of GDNF that has Functional Activity that is the subject of a Subsequent Distracting Program.

**SECTION 1.74** **"Subsequent Distracting Program"** means, during the applicable period specified in Article 8 (Termination), the Exploitation of any Gene Therapy product that delivers GDNF or the gene encoding GDNF, or in either case any fragment of GDNF that has Functional Activity.

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**SECTION 1.75** **"Subsequent Distracting Transaction"** means, during the applicable period specified in Section 8.5.1.3, any transaction whereby a Third Party that is engaged in a Subsequent Distracting Program becomes an Affiliate or is merged with AMT during the applicable period.

**SECTION 1.76** **"Taxes"** has the meaning set forth in Section 6.4.1 (Payment of Taxes).

**SECTION 1.77** **"Third Party"** means a Person other than (i) AMT or any of its Affiliates, and (ii) Amgen or any of its Affiliates.

**SECTION 1.78** **"Third Party License Payments"** means (i), with respect to any agreement that relates to intellectual property rights that solely Cover a GDNF Product, any payments made by AMT and/or its Affiliates (whether up-front payments, milestones, royalties or research funding (provided that with respect to research funding payments, only the amounts in excess of AMT's external costs and internal costs directly related to such research activities shall be included) during the term of this Agreement to any Third Party for intellectual property rights that are necessary in the Exploitation of GDNF Products (but solely with respect to intellectual property rights that would be infringed by the Exploitation of GDNF Products absent a license therefore) including but not limited to payments to the National Institutes of Health and/or the Food and Drug Administration, and (ii), with respect to any agreement that relates to intellectual property rights that Cover more than solely a GDNF Product, any royalty payments made by AMT and/or its Affiliates during the Payment Term to any Third Party for intellectual property rights that are necessary in the Exploitation of GDNF Products. For the sake of clarity, Third Party License Payments specifically excludes any payments that are related to any device used in connection with the delivery of a GDNF Product.

**SECTION 1.79** **"Unpartnered Net Revenues"** means any Net Revenue other than Partnered Net Revenues.

**SECTION 1.80** **"U.S."** or **"United States"** means the several States of the United States of America, the District of Columbia, and the commonwealths, territories, and possessions of the United States of America.

**SECTION 1.81** **"VAT"** has the meaning set forth in Section 6.4.3 (VAT).

## **ARTICLE 2. LICENSES**

**SECTION 2.1** **License to AMT.** Subject to Section 2.2, Amgen hereby grants to AMT and its Affiliates, during the Payment Term, (i) a worldwide, exclusive (even as to Amgen) license, with the right to grant Sublicenses, under the Licensed Patent Rights to Exploit GDNF Products in the Licensed Field, and (ii) a worldwide, exclusive (even as to Amgen) license, with the right to grant Sublicenses, under the Licensed Know-How to Exploit GDNF Products in the

Licensed Field. Upon expiration of the Payment Term in a giving country, Amgen hereby grants to AMT and its Affiliates (i) a worldwide, non-exclusive license, with the right to grant Sublicenses, under the Licensed Patent Rights to Exploit GDNF Products in the Licensed Field, and (ii) a worldwide,

non-exclusive license, with the right to grant Sublicenses, under the Licensed Know-How to Exploit GDNF Products in the Licensed Field.

**SECTION 2.2**      **Retention of Rights.** Notwithstanding anything herein to the contrary, Amgen retains (on behalf of itself and its Affiliates) a non-exclusive license under the Licensed Know-How and Licensed Patent Rights to: (i) conduct research (including preclinical and non-clinical activities and including academic collaborations, but excluding in vivo and clinical activities) with respect to, but not to develop or commercialize, GDNF Products in the Licensed Field; and (ii) use, make and have made GDNF Products for the purposes of such research. Additionally, notwithstanding anything herein to the contrary, Amgen retains (on behalf of itself and its Affiliates) the right to Exploit, and authorize (by license or otherwise) Third Parties to Exploit, GDNF outside the Licensed Field. No more than [\*\*] during the Term, AMT may ask Amgen in writing whether, during the period from the last request (or if there has not been such a request, from the Effective Date) Amgen is or has been a party to an academic collaboration with respect to GDNF Products in the Licensed Field. Amgen shall reply to AMT within [\*\*] days of any such request by AMT and if in such reply Amgen notifies AMT that Amgen is or has been a party to such a collaboration, Amgen shall also inform AMT whether any inventions have been created, developed or reduced to practice in the course of such collaboration, the identity and nature of such inventions and whether Amgen owns or has taken an exclusive license to such inventions. If Amgen does not own or has not taken an exclusive license to such inventions then AMT may request Amgen to use good faith efforts to obtain an exclusive license to such inventions (to the extent Amgen has the contractual right to do so) for the benefit of AMT (provided that AMT shall be solely and directly liable for any amounts payable to the academic collaborator in relation to such license). Amgen represents and warrants that, as of the Effective Date, it owns or has been granted a license to all inventions with respect to GDNF Products in the Licensed Field that have been created, developed or reduced to practice in the course of any academic collaboration to which Amgen is or has been a party.

**SECTION 2.3**      **Transfer of Licensed Know-How.** AMT hereby acknowledges that Amgen has completed its obligation to transfer Know-How and Materials to AMT under the Original Agreement and Amgen has no unfulfilled obligations to transfer any Know-How or Materials to AMT. AMT acknowledges that the Materials transferred by Amgen to AMT under the Original Agreement are experimental in nature and may have unknown characteristics and therefore agrees to use prudence and reasonable care in the use, handling, storage, transportation and disposition and containment of any such Materials. Accordingly, no such Materials (unless expressly provided by Amgen otherwise in writing) shall be used in any human application, including any clinical trial.

**SECTION 2.4**      **No Other Rights.** AMT acknowledges that the rights and licenses granted under this Article 2 (Licenses) and elsewhere in this Agreement are limited to the scope expressly granted. Accordingly, except for the rights expressly granted under this Agreement, no right, title, or interest of any nature whatsoever is granted whether by implication, estoppel, reliance, or otherwise, by Amgen to AMT. All rights that are not specifically granted herein are reserved to Amgen. Without limiting the foregoing, AMT hereby acknowledges that Amgen retains the right

to Exploit, and authorize (by license or otherwise) Third Parties to Exploit, any product other than a GDNF Product even if such product is covered by a claim within the Licensed Patent Rights. For the sake of clarity, Amgen has granted certain rights under the Licensed Know-How and Licensed Patent Rights to MedGenesis Therapeutix Inc. and Biovail Laboratories International SRL outside of the Licensed Field.

**SECTION 2.5**      **Limited Exploitation Rights.** Without limiting the provisions of Section 2.4 (No Other Rights), AMT agrees (on behalf of itself and its Affiliates), and shall cause each of its Sublicensees to agree as a condition to the grant of a Sublicense, not to Exploit any Licensed Know-How or Licensed Patent Rights in any use outside the Licensed Field or for any products other than GDNF Products.

**SECTION 2.6**      **Collaboration Option.** AMT shall notify Amgen in writing within [\*\*] Business Days after Completion of the first Phase 2 Clinical Trial (whether a stand-alone clinical trial or in combination with another clinical trial, i.e., a phase 1/2 clinical trial or phase 2/3 clinical trial) for the first GDNF Product. Amgen shall have [\*\*] Business Days from receipt of such notice to request from AMT a data package containing such information as Amgen may reasonably request and as may be available regarding such GDNF Product (the “**Data Package**”). In the event that Amgen requests such Data Package, then Amgen shall have the right, by giving notice in writing to AMT, to exercise an option to obtain an exclusive license from AMT to Exploit GDNF Products in the Collaboration Territory (the “**Collaboration Option**”) (such option to expire [\*\*] Business Days after the later of (i) receipt of the complete Data Package and (ii) a face-to-face meeting between the Parties to discuss any data contained in the Data Package (provided that such meeting is held within [\*\*] Business Days after receipt of the complete Data Package). In the event Amgen exercises the Collaboration Option, then the Parties shall enter exclusive good faith negotiations for a period of [\*\*] Business Days (from the date of Amgen’s notice exercising the Collaboration Option) with respect to a definitive agreement governing the respective rights and obligations of the Parties with respect thereto. In the event that the Parties are unable to enter into a definitive agreement within such [\*\*] Business Day period, then AMT shall have the right to retain its development and commercialization rights in the Collaboration Territory; **provided, however**, that AMT would not be entitled to subsequently grant development or commercialization rights in the Collaboration Territory to a Third Party on financial terms less favorable to AMT than those last offered by Amgen.

## **ARTICLE 3.      MANUFACTURING, REGULATORY AND COMMERCIAL ACTIVITIES**

**SECTION 3.1**      **Manufacturing.** AMT, itself or through one or more Third Party contract manufacturers designated by it that have appropriate expertise and are suitably qualified, including holding the requisite permits and having been subject to or available for appropriate audits and inspections by the relevant regulatory bodies solely responsible for the manufacture and supply of requirements of GDNF Products (including any GDNF used in a GDNF Product). In addition to the CMOs listed on Schedule 3.1, AMT may, with the prior written consent of Amgen, such consent not to be unreasonably withheld or delayed, transfer the Licensed Know-How related to

the manufacture of GDNF to one or more other Third Party contract manufacturers at AMT’s own expense, under obligations of confidentiality and limitations on use consistent with those set out in this Agreement. From time to time upon the written request of Amgen (but not more than [\*\*]), AMT shall deliver to Amgen a written summary of manufacturing activities conducted hereunder by it since the last such report. In the event that Amgen exercises the Collaboration Option and all other terms of a collaboration agreement are agreed by the Parties within the time period specified in Section 2.6 (Collaboration Option), AMT would agree,

upon Amgen's request, to supply Amgen with clinical and commercial GDNF Product for use in the Collaboration Territory at AMT's cost as determined on an IFRS basis.

**SECTION 3.2**      **Regulatory Process.** Subject to the terms of any collaboration agreement entered into by the Parties in accordance with Section 2.6 (Collaboration Option), AMT shall have sole and full control, authority, discretion and right to conduct (by itself, through its Affiliates or via a Third Party) and make all decisions regarding all development and regulatory matters (including interaction with all Governmental Authorities and preparation of any Regulatory Filings) with respect to GDNF Products. From time to time upon the written request of Amgen (but not more than [\*\*] in any Calendar Year), AMT shall deliver to Amgen a written summary of development and regulatory activities conducted hereunder by it since the last such report. Amgen shall grant a right of reference to AMT to all Regulatory Approvals and Regulatory Filings owned by Amgen as of the Effective Date, if any, relating to GDNF Products in the Licensed Field. All Regulatory Approvals and Regulatory Filings obtained by AMT relating to GDNF Products in the Licensed Field shall be solely owned by AMT and held in the name of AMT or its respective designated Affiliates or Sublicensee(s).

**SECTION 3.3**      **Commercial Activities.** Subject to the terms of any collaboration agreement entered into by the Parties in accordance with Section 2.6 (Collaboration Option), AMT shall have sole responsibility for commercialization of GDNF Products. AMT shall be solely responsible for all costs incurred in its commercialization of GDNF Products. From time to time upon the written request of Amgen (but not more than [\*\*] in any Calendar Year), AMT shall deliver to Amgen a written summary of commercialization activities conducted hereunder by it since the last such report; provided that nothing in this Section 3.3 (Commercial Activities) shall require AMT to disclose the identity of any current or prospective customer. AMT shall have sole discretion with respect to the pricing of GDNF Products.

#### **ARTICLE 4.      FEES AND REVENUE SHARING PAYMENTS**

**SECTION 4.1**      **Milestone Fee.** Upon the first approval of an NDA with respect to a GDNF Product achieved by AMT and/or its Affiliates (the "**Approval Milestone**"), AMT shall promptly (and, in any event, no later than [\*\*] days) notify Amgen in writing of such occurrence and the amount received, and Amgen will invoice AMT for a one-time payment in the amount of the greater of (a) ten million US Dollars (US\$10,000,000) and (b) [\*\*]% of any milestone payments received by AMT from Third Parties in respect of the Approval Milestone. Such payment shall be due and payable by AMT within [\*\*] days after receipt of the invoice from Amgen. The milestone

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fee is payable only once and shall not be payable for subsequent or repeated achievements of such milestone event with one or more of the same or different GDNF Products.

#### **SECTION 4.2**      **Revenue Sharing Payments.**

During the Payment Term, AMT shall make payments ("**Revenue Sharing Payments**") to Amgen equal to:

- (i) [\*\*] percent ([\*\*]%) of any and all Unpartnered Net Revenues received by AMT and/or its Affiliates (but not by its Sublicensees) in any Calendar Quarter, less:
  - (a) COGS attributable to the GDNF Product sold or used in connection with the applicable service performed in such Calendar Quarter;
  - (b) any patent prosecution and maintenance costs incurred by AMT in the event that it has assumed responsibility to prosecute and maintain any of the Licensed Patent Rights in accordance with Section 7.1 (Patent Prosecution); and
  - (c) any and all Third Party License Payments that are payable by AMT and/or any of its Affiliates in such Calendar Quarter ((a), (b), and (c) together being "**Allowable Offsets**").

For the purpose of calculating Unpartnered Net Revenues for any given Calendar Quarter the maximum deduction for all Allowable Offsets taken together in a Calendar Quarter shall be [\*\*] percent ([\*\*]%) (the "**Floor**") of the total applicable Unpartnered Net Revenues for that Calendar Quarter. If the existence of the Floor results in AMT not being able to deduct the full amount of any Allowable Offsets in the Calendar Quarter when such deduction first becomes applicable then AMT can roll forward the outstanding amount to the next Calendar Quarter;

- (ii) [\*\*] percent ([\*\*]%) of any and all Sublicensing Revenues received by AMT and/or its Affiliates in any Calendar Quarter; and
- (iii) [\*\*] percent ([\*\*]%) of any and all Partnered Net Revenues received by AMT and/or its Sublicensee (and/or their respective Affiliates) in any Calendar Quarter, less:
  - (a) COGS attributable to the GDNF Product sold or used in connection with the applicable service performed in such Calendar Quarter;
  - (b) any patent prosecution and maintenance costs incurred by AMT in the event that it has assumed responsibility to prosecute and maintain any of the Licensed Patent Rights in accordance with Section 7.1 (Patent Prosecution); and

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- (c) any and all Third Party License Payments that are payable by AMT and/or any of its Affiliates in such Calendar Quarter ((a), (b), and (c) together being "**Allowable Offsets**").

For the purpose of calculating Partnered Net Revenues for any given Calendar Quarter the maximum deduction for all Allowable Offsets taken together in a Calendar Quarter shall be [\*\*] percent ([\*\*]%) (the "**Floor**") of the total applicable Partnered Net Revenues for that Calendar Quarter. If the existence of the Floor results in AMT not being able to deduct the full amount of any Allowable Offsets in the Calendar Quarter when such deduction first becomes applicable then AMT can roll forward the outstanding amount to the next Calendar Quarter.

#### **ARTICLE 5.      DILIGENCE**

**SECTION 5.1** Use of Reasonably Diligent Efforts. AMT shall use Reasonably Diligent Efforts to develop at least one GDNF Product, and seek to obtain Regulatory Approvals for such GDNF Product in the U.S. and the European Union, and commercialize such GDNF Product.

**SECTION 5.2** Sequential Implementation. The Parties acknowledge that developing and seeking Regulatory Approvals for a GDNF Product may include sequential implementation of clinical trials and intervals between clinical trials for data interpretation and clinical program planning and approval.

**SECTION 5.3** Holistically Determined. Amgen acknowledges that in determining whether AMT has met its requirement to use Reasonably Diligent Efforts in accordance with Section 5.1 (Use of Reasonably Diligent Efforts) reference will be made to the totality of AMT's activities with respect to all GDNF Product(s) (if, in AMT's sole discretion, it decides to develop more than one GDNF Product) in all countries, not on a country-by-country basis.

**SECTION 5.4** Excluded from Consideration. Notwithstanding Section 5.3 (Holistically Determined), in determining whether AMT has used Reasonably Diligent Efforts, Reasonably Diligent Efforts shall be determined as if no payments were required to be made by AMT pursuant to Article 4 (Fees and Revenue Sharing Payments) of this Agreement.

**SECTION 5.5** Diligence Schedule. In addition to the obligations of AMT to use Reasonably Diligent Efforts set forth above, AMT shall commence a proof of concept study in non-human primates ("**POC Study**") within [\*\*] months from the Effective Date ("**Diligence Period**"), provided however that, where there is a study plan, budget and agreement in place for the POC Study but such POC Study has not commenced within the Diligence Period because there are insufficient animals available that are suitable for use in such study, for example due to elevated anti-AAV antibody titre levels, the Diligence Period shall be extended until such animals are available. During this extension period, AMT shall use Reasonably Diligent Efforts to ensure the availability of such animals as soon as practicably possible. If the POC Study has not commenced within the initial [\*\*] month Diligence Period, AMT shall promptly (but in no event later than [\*\*] days after the end of such initial Diligence Period) notify Amgen in writing of such failure to

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achieve such event within the Diligence Period (the "**Diligence Notice**") and if such failure is due to the lack of animals then AMT shall provide details thereof and the Diligence Period shall be extended. If the failure of AMT to commence the POC Study within [\*\*] months from the Effective Date is due to factors other than lack of animals, Amgen shall have the right to terminate the Agreement by providing written notice to AMT no later than twenty (20) Business Days after receipt of the Diligence Notice.

## **ARTICLE 6. REPORTS, PAYMENTS AND RECORDS**

**SECTION 6.1** Payment; Reports. During the Payment Term, AMT will prepare and deliver to Amgen Revenue Sharing Payments and reports supporting such Revenue Sharing Payments (as described below) for each Calendar Quarter within [\*\*] days of the end of each such Calendar Quarter. Each Revenue Sharing Payment will be made from a single account in the country in which AMT is headquartered accompanied by a report of (i) Net Revenues of GDNF Products stating: (a) the aggregate gross invoiced sales prices charged for GDNF Products and the aggregate revenues from any use of a GDNF Product in the provision of a service performed; (b) Net Sales and Net Service Revenues of GDNF Products during the applicable Calendar Quarter; (c) COGS related to units sold and services provided in such Calendar Quarter; and (ii) the amount and type of payment received from each Sublicensee during the applicable Calendar Quarter and (iii) any and all Third Party License Payments that are payable by AMT and/or any of its Affiliates in such Calendar Quarter (a "**Payment Report**"). From time to time, Amgen may change its accounting and financial reporting practices from Calendar Quarters and Calendar Years to fiscal quarters and fiscal years or vice versa. If Amgen notifies AMT of a change in Amgen's accounting and financial reporting practices from Calendar Quarters and Calendar Years to fiscal quarters and fiscal years or vice versa, then thereafter, beginning with the period specified in the notice, the payment, reporting and other obligations of AMT hereunder related to Calendar Quarters and Calendar Years shall be deemed satisfied by compliance therewith in accordance with the new reporting periods (fiscal reporting periods or calendar reporting periods, as the case may be) instead of the previously utilized reporting periods.

**SECTION 6.2** Invoices. To the extent an invoice is required to be submitted by Amgen to AMT, such invoice shall be addressed to:

AMT  
FAO Finance Department  
P.O. Box 22506  
1100 DA Amsterdam  
The Netherlands

Invoices not submitted to the foregoing address may be subject to delay or return.

**SECTION 6.3** Exchange Rate; Manner and Place of Payment. With respect to Net Revenues invoiced or expenses incurred in a currency other than U.S. dollars or Sublicensing Revenue received in a currency other than U.S. dollars (each a "**Foreign Currency Amount**"), the Foreign

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Currency Amount shall be converted into the U.S. dollar equivalent using a rate of exchange which corresponds to the rate used by AMT, for the respective reporting period, related to recording such Foreign Currency Amount in its books and records that are maintained in accordance with IFRS (**provided, however**, that if, at such time, AMT does not use a rate for converting into a U.S. dollar equivalent that is maintained in accordance with IFRS, then AMT will use a rate of exchange which corresponds to the rate of exchange for such currency reported in The Wall Street Journal, Internet U.S. Edition, as of the last day of the applicable reporting period (or, if unavailable on such date, the first date thereafter on which such rate is available)). Any payment of royalties or Revenue Sharing Payment shall be calculated based upon the U.S. dollar equivalent calculated in accordance with the foregoing. Any Payment Report required under Section 6.1 (Payment; Reports) shall provide the details and background information used to calculate such currency conversion. All payments shall be made in U.S. dollars.

Payment of all sums due hereunder shall be made by check, wire transfer, or electronic funds transfer (EFT), at Amgen's choice, to the following:

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**SECTION 6.4** Taxes.



- 6.4.1 Payment of Taxes. All excises, taxes, and duties, excluding any value-added tax (collectively “**Taxes**”) levied on account of a payment made by AMT to Amgen pursuant to this Agreement will be the responsibility of and paid by Amgen or shall be subject to the withholding, remittance, and offset provisions of this Section 6.4 (Taxes).
- 6.4.2 Withholding by AMT. In the event that Law requires AMT to withhold Taxes with respect to any payment to be made by AMT pursuant to this Agreement, AMT will withhold such Taxes from the amount due and furnish, within [\*\*] days of the date of such withholding, Amgen with proof of payment of such Taxes. AMT will provide reasonable assistance to Amgen in its efforts to claim an exemption of Taxes, obtain a refund of Taxes withheld, or obtain a credit with respect to such Taxes paid. In order for Amgen to secure an exemption from, or a reduction in, any withholding of Taxes, Amgen shall provide to AMT such forms as reasonably required for each type of payment to be made pursuant to the Agreement for which an exemption from, or a reduction in, any withholding of Taxes is claimed. In the event that a form previously furnished to AMT expires, is incorrect, or is inapplicable to the type of payment to be made, due to a change in circumstances or otherwise, Amgen shall furnish a new form to AMT prior to the payment of any such amount in order to secure an exemption from, or a reduction in, any withholding of Taxes with respect to such payment.
- 6.4.3 VAT. All payments due to Amgen from AMT pursuant to this Agreement shall be paid exclusive of any value-added tax (“**VAT**”) (which, if applicable, shall be payable by AMT upon receipt of a valid VAT invoice).

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SECTION 6.5 Audits. AMT shall keep (and cause its Affiliates and Sublicensees to keep) true and fair records of the underlying revenue and expense data relating to the calculations of Net Revenues and Revenue Sharing Payments, as well as any other payments required under this Agreement. Amgen shall have the right, at its own expense and not more than [\*\*] during the term of this Agreement, to have an independent, certified public accountant, selected by Amgen, audit the records of AMT, its Affiliates and Sublicensees in the location(s) where such records are maintained by the applicable entity upon reasonable notice (which shall be no less than [\*\*] days prior written notice) and during regular business hours, and for the sole purpose of verifying the basis and accuracy of payments required and made under this Agreement. To the extent that AMT does not have the right to grant Amgen the right to audit its Sublicensees’ books and records hereunder, AMT shall obtain for itself such right and, at the request of Amgen, AMT shall exercise such audit right with respect to Sublicensees and provide the results of such audit for inspection by Amgen pursuant to this Section 6.5 (Audits). The books and records for any particular Calendar Year shall only be subject to [\*\*]. The report and communication of such accountant with respect to such an audit shall be limited to a certificate stating whether any report made or payment submitted by AMT during such audited period is accurate or inaccurate and the amount of any payment discrepancy. Such accountant shall provide Amgen and AMT with a copy of each such report simultaneously. Should the audit lead to the discovery of a discrepancy to Amgen’s detriment, AMT shall pay the amount of the discrepancy within [\*\*] days of AMT’s receipt of the report. Additionally, in the event that the discrepancy is to Amgen’s detriment and is greater than [\*\*] percent ([\*\*]%) of all payments due in such audited period, then AMT shall pay interest on such amount at an annual rate of the [\*\*]. Should the audit lead to the discovery of a discrepancy to AMT’s detriment, AMT will have the right to deduct such amount from any future payment obligations. Amgen shall pay the full cost of the audit unless the discrepancy is to Amgen’s detriment and is greater than [\*\*] percent ([\*\*]%) of all payments due in such audited period, in which case AMT shall pay or reimburse the reasonable cost charged by such accountant for such audit. Upon the expiration of [\*\*] years following the end of any Calendar Year, the right to audit the books and records for such Calendar Year shall expire and the calculation of payments payable with respect to each such Calendar Year shall be binding and conclusive upon Amgen and AMT, its Affiliates and Sublicensees shall be released from any liability or accountability with respect to payments for such Calendar Year. AMT shall no longer be required to retain such records for such Calendar Year after the expiration of such [\*\*] year period.

SECTION 6.6 Confidentiality. Amgen shall treat all financial information subject to review under this Article 6 (Reports, Payments and Records), or under any related Sublicense, as AMT’s Confidential Information.

## **ARTICLE 7. PATENT PROSECUTION**

SECTION 7.1 Patent Prosecution. As between the Parties, Amgen shall have the sole right, at Amgen’s sole expense, to control the Prosecution and Maintenance of the Licensed Patent Rights, including, if Amgen so elects, through the use of outside counsel. Solely with respect to Licensed Patent Rights covering the GDNF Products in whole or in part in the Licensed Field, Amgen agrees to: (i) keep AMT reasonably informed with respect to such activities; and (ii) provide AMT

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with all material documentation and correspondence from, sent to or filed with patent offices regarding the Licensed Patent Rights. If Amgen (or its designee) determines to abandon any claims within the Licensed Patent Rights that have application solely in the Licensed Field, then Amgen shall provide AMT with notice at least [\*\*] days prior to the date such abandonment would become effective. In such event, subject to any right of a Third Party as of the Effective Date to control Prosecution and Maintenance of the Licensed Patent Rights, AMT shall have the right, at its option, to control the Prosecution and Maintenance of such claims, at its own expense (subject to Section 4.2(i) and 4.2(iii)), in Amgen’s name. For purposes of the foregoing, “**Prosecution and Maintenance**” means, with respect to a patent or patent application, the preparing, filing, prosecuting and maintenance of such patent or application, as well as re examinations, reissues, requests for patent term extensions and the like with respect thereto, together with the conduct of interferences, the defense of oppositions and other similar proceedings with respect to the particular patent or patent application.

SECTION 7.2 Defense of Third Party Infringement Claims. If any GDNF Product Exploited by or under the authority of AMT becomes the subject of a Third Party’s claim or assertion of infringement of a patent relating to the manufacture, use, sale, offer for sale or importation of such GDNF Product, the Party first having notice of the claim or assertion shall promptly notify the other Party, and the Parties shall promptly confer to consider the claim or assertion and the appropriate course of action. Unless the Parties otherwise agree in writing, each Party shall have the right to defend itself against a suit that names it as a defendant (the “**Defending Party**”). Neither Party shall enter into any settlement of any claim described in this Section 7.2 (Defense of Third Party Infringement Claims) that admits to the invalidity or unenforceability of the Licensed Patent Rights, incurs any financial liability on the part of the other Party or requires an admission of liability, wrongdoing or fault on the part of the other Party without such other Party’s written consent, which consent shall not be unreasonably withheld or delayed. In any event, the other Party shall reasonably assist the Defending Party and cooperate in any such litigation at the Defending Party’s request and expense.

## SECTION 7.3 Enforcement.

7.3.1 Subject to the provisions of this Section 7.3 (Enforcement), in the event that a Party reasonably believes that any Licensed Patent Right is being infringed by a Third Party or is subject to a declaratory judgment action or impeachment action (or any other similar judicial court proceeding) arising from such infringement, in each case with respect to the manufacture, use, sale, offer for sale or importation of a GDNF Product in the Licensed Field (an

“**Infringing Product**”), such Party shall promptly notify the other Party. In such event, AMT shall have the initial right (but not the obligation) to enforce such Licensed Patent Rights in the Licensed Field with respect to infringement by an Infringing Product, or to defend any declaratory judgment action or impeachment action (or defend any other similar judicial court proceeding) with respect thereto (an “**Enforcement Action**”), at AMT’s expense. Amgen shall join, at AMT’s expense, as a party plaintiff if required by applicable Law to maintain the action and, in any event, shall, at AMT’s expense, give AMT reasonable assistance and authority to file and

prosecute such Enforcement Action. If, in an Enforcement Action initiated by AMT, Amgen is involuntarily joined other than by AMT, then AMT shall bear Amgen’s expenses for the prosecution of the Enforcement Action. In the event that any Licensed Patent Right covering both the Licensed Field and activity outside the Licensed Field is being infringed by a Third Party or is subject to a declaratory judgment action, impeachment action or any other challenge to the Licensed Patent Rights, then, subject to Section 7.3.4, upon AMT’s written request, Amgen shall use good faith reasonable efforts to gain the cooperation of any licensee of the Licensed Patent Rights for use outside of the Licensed Field in connection with the enforcement with respect to such infringement or with respect to the defense of any such declaratory judgment action, impeachment action or other challenge.

7.3.2 In the event that AMT fails to initiate an Enforcement Action to enforce such Licensed Patent Rights against such an Infringing Product in the Licensed Field, within [\*\*] days of a request by Amgen to do so (or such shorter period of time as necessary to prevent the loss of Amgen’s (or its designee’s) ability to subsequently initiate an Enforcement Action or diminution of rights or remedies thereunder (whether due to an expiration of the statute of limitations, estoppel by laches or similar doctrine)), Amgen (or its designee) may initiate an Enforcement Action against such infringement at its own expense. The Party initiating or defending any such Enforcement Action (the “**Enforcing Party**”) shall keep the other Party reasonably informed of the progress of any such Enforcement Action, and such other Party shall have the right to participate with counsel of its own choice at its own expense. In any event, the other Party shall reasonably cooperate with the Enforcing Party, including providing information and materials, at the Enforcing Party’s request and expense.

7.3.3 Any recovery received as a result of any Enforcement Action to enforce Licensed Patent Rights in the Licensed Field pursuant to this Section 7.3 (Enforcement) shall be used first to reimburse the Parties for the costs and expenses (including attorneys’ and professional fees) incurred in connection with such Enforcement Action (and not previously reimbursed), and the remainder of the recovery shall be shared as follows:

7.3.3.1 If AMT is the Enforcing Party: (i) with respect to such portion of the recovery that is characterized as lost profits in the Licensed Field, [\*\*] percent ([\*\*]%) to AMT (provided, however, that such portion of the recovery will be used to calculate Net Revenues attributable to such lost profits (and AMT shall pay Amgen Revenue Sharing Payments on such (hypothetical) Net Revenues in accordance with Section 4.2(i)); and (ii) with respect to such portion of the recovery that is not characterized as lost profits, [\*\*] percent ([\*\*]%) to AMT;

7.3.3.2 If Amgen or its designee is the Enforcing Party, [\*\*] to Amgen and [\*\*] to AMT; and

7.3.3.3 In all cases, any such remainder which is a recovery from such an Enforcement Action which represents damages from sales of products other than Infringing Products or

outside the Licensed Field (e.g. sales of a product outside the Licensed Field) shall inure solely and be paid over to Amgen.

7.3.4 For clarity, except as may be otherwise agreed between the Parties in writing, as between the Parties, Amgen shall have the exclusive right, at its expense, to control the enforcement of or defend any declaratory judgment action with respect to the Licensed Patent Rights outside the Licensed Field and shall retain all recoveries arising therefrom. Amgen shall notify AMT of any such action, and, if Amgen (rather than a licensee of Amgen) is directly handling such action, then Amgen shall keep AMT reasonably informed of the progress of any such action and reasonably consider any comments AMT may have with respect to such action.

7.3.5 Neither Party shall enter into any settlement of any claim described in this Section 7.3 (Enforcement) that admits to the invalidity or unenforceability of the Licensed Patent Rights, incurs any financial liability on the part of the other Party or requires an admission of liability, wrongdoing or fault on the part of the other Party without such other Party’s written consent, which consent shall not be unreasonably withheld or delayed. In any event, the other Party shall reasonably assist the Enforcing Party and cooperate in any such litigation at such Enforcing Party’s request and expense.

**SECTION 7.4** Patent Term Extensions/Supplementary Protection Certificates. Subject to any rights of a licensee of Amgen existing as of the Effective Date under any Licensed Patent Rights (a “**Pre-Existing Licensee**”), Amgen hereby authorizes AMT, its Affiliates and Sublicensees to seek patent term extensions and supplementary protection certificates (collectively, “**PTEs**”) of any or all Licensed Patent Rights in all countries throughout the world where such rights are obtainable. Prior to seeking any such PTEs, AMT shall notify Amgen in writing of its intent to seek a PTE (a “**PTE Notice**”). Amgen shall notify AMT in writing within [\*\*] days of receipt of a PTE Notice of whether AMT is permitted to seek such a PTE or whether such PTE would be reasonably likely to conflict with a Pre-Existing Licensee’s right to seek any similar patent term extensions and supplementary protection certificates. Amgen shall reasonably cooperate with AMT’s efforts to file applications for such patent term extensions and supplementary protection certificates; *provided, however*, that AMT shall reimburse Amgen for its reasonable out-of-pocket expenses with respect to such cooperation.

**SECTION 7.5** Patent Marking. AMT shall mark (or caused to be marked) all GDNF Products marketed and sold hereunder with appropriate Licensed Patent Rights numbers or indicia at Amgen’s request to the extent permitted by Law, in those countries in which such notices impact recoveries of damages or remedies available with respect to infringements of patents.

## **ARTICLE 8. TERMINATION**

**SECTION 8.1** Term. The term of this Agreement shall commence on the Effective Date, and, unless sooner terminated, continue in perpetuity.

## SECTION 8.2      Termination by AMT.

- 8.2.1 Termination At Will. AMT shall have the right to terminate this Agreement at any time by giving Amgen ninety (90) days' written notice of AMT's election to terminate this Agreement.
- 8.2.2 Default. Upon any material breach of this Agreement by Amgen, AMT, in addition to any other remedy available at law or equity, shall have the right to provide notice to Amgen of such material breach. In the event such material breach has not been cured by Amgen within [\*\*] days after receipt of such notice, then, AMT shall have the right to terminate this Agreement by providing Amgen with [\*\*] days written notice *provided, however*, that if such breach is not susceptible of cure within the stated [\*\*] day period and Amgen uses diligent, good faith efforts to cure such breach, the stated period will be extended by an additional [\*\*] days.

## SECTION 8.3      Termination by Amgen.

- 8.3.1 Monetary Default. Upon any failure by AMT to timely pay undisputed amounts due to Amgen required to be paid under this Agreement, Amgen, in addition to any other remedy available at law or equity, shall have the right to terminate this Agreement by giving AMT thirty (30) days' prior written notice. This Agreement shall terminate upon the expiration of such thirty (30) day period, unless on or before such date AMT has paid such amounts (including the payment of interest on such amounts at an annual rate of the [\*\*], in which case the Agreement shall continue unaffected.
- 8.3.2 Diligence Default. Amgen shall have the right to terminate the Agreement in accordance with Section 5.5 (Diligence Schedule).
- 8.3.3 Nonmonetary Default. Upon any material non-monetary breach of this Agreement by AMT (*i.e.*, not involving the payment by AMT of any amounts required to be paid under this Agreement), Amgen, in addition to any other remedy available at law or equity, shall have the right to provide notice to AMT of such material non-monetary breach. In the event such material non-monetary breach has not been cured by AMT within [\*\*] days after receipt of such notice, then, Amgen shall have the right to terminate this Agreement by providing AMT with [\*\*] days written notice *provided, however*, that if such breach is not susceptible of cure within the stated [\*\*] day period and AMT uses diligent, good faith efforts to cure such breach, the stated period will be extended by an additional [\*\*] days.

## SECTION 8.4      Effects of Termination of this Agreement.

- 8.4.1 Sublicenses. Save as expressly provided in Section 8.4.3 and Section 8.5 (Reversion Rights), termination of this Agreement for any reason shall effect the immediate and simultaneous termination of all Sublicenses and of all rights extended to the Affiliates of AMT subject to the provisions of Section 8.4.2 (GDNF Product in Stock).

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- 8.4.2 GDNF Product in Stock. For a period of [\*\*] days after the termination of this Agreement under either Section 8.2 (Termination by AMT) or 8.3 (Termination by Amgen), AMT, its Affiliates and Sublicensees shall have the right to sell off any stock of GDNF Product on hand or in-process and provide services relating to GDNF Products, and the provisions of Section 4.2 (Revenue Sharing) and the payment provisions of this Agreement shall apply to such sales.
- 8.4.3 Sublicensees. In the event of the termination of this Agreement under Section 8.3 (Termination by Amgen), Amgen will, if required by AMT (and provided that the applicable Sublicensee is not in breach of any Sublicense granted to it under this Agreement), enter into direct licenses with up to no more than [\*\*] Sublicensees of AMT on terms which are the same as or substantially similar to those set out in this Agreement; provided, however, that the request shall be made once and all Sublicensed territories throughout the world must be covered by the aggregate direct license agreements.

## SECTION 8.5      Reversion Rights.

- 8.5.1 At Will or For AMT's Breach. In the event that AMT terminates the Agreement under Section 8.2.1 (Termination At Will) or Amgen terminates the Agreement under Section 8.3.1 or 8.3.3 (but not under Section 8.3.2), then, the following provisions shall apply:
- 8.5.1.1 Grant of License. Upon Amgen's written request, AMT grants Amgen and its Affiliates a worldwide, royalty-bearing, exclusive license, with the right to grant Sublicenses, under the AMT Licensed Patent Rights and AMT Licensed Know-How to Exploit GDNF Products in the Licensed Field on the terms of Articles 4.6, 8.2.1, 8.3.1 and 8.3.3 of this Agreement such that the term "AMT" shall be replaced by the term "Amgen" and the term "Amgen" by the term "AMT" save that the Revenue Sharing Payments due to AMT shall be [\*\*] percent ([\*\*]%) for payments under Section 4.2(i), [\*\*] percent ([\*\*]%) for payments under Section 4.2(iii), and [\*\*] percent ([\*\*]%) for payments under Section 4.2(iii). In addition, the provisions of Section 9.5 shall apply to the AMT Licensed Know-How save that Amgen's obligations in respect of the AMT Licensed Know-How shall apply for [\*\*] years post termination (rather than [\*\*] years post termination). In the event that any intellectual property rights that are useful or necessary to Exploit GDNF Products are not included in the foregoing license grant by reason of a requirement on the part of AMT under a written agreement, including any patents licensed to AMT by the National Institutes of Health and/or the FDA, (a "**Third Party Agreement**") to pay a Third Party any amounts with respect to Amgen's Exploitation of a GDNF Product, Amgen shall have the right, but not the obligation, to elect to obtain a license under such Third Party intellectual property rights. In the event Amgen elects to obtain such a license, Amgen shall provide written notice thereof to AMT. If Amgen so elects, then each Party shall use Reasonably Diligent Efforts to obtain such a direct license to Amgen from such Third Party provided that such license is on substantially similar terms to the Third Party Agreement and in any event on

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financial terms that are no less favourable to Amgen than under the Third Party Agreement. If it is not possible to obtain such a direct license to Amgen then AMT shall use Reasonably Diligent Efforts to grant a sublicense under the Third Party Agreement to Amgen, to the extent that such sublicensing is allowed by the Third Party Agreement. If sublicensing under any Third Party Agreements is allowed but limited or restricted, AMT shall, upon request by Amgen, seek consent or take such other actions as are reasonably necessary to seek to enable AMT to sublicense under such Third Party Agreements. In the event that Amgen elects to obtain a sublicense, then Amgen shall pay AMT any amounts owed to such Third Party under the Third Party Agreements by reason of Amgen's Exploitation of GDNF Products. Amgen shall indemnify AMT on the terms of Section 8.9.1 in respect of any Third Party (including any licensor) claims relating to Amgen's performance under the sublicense. Amgen shall have

the right, in its sole discretion, to terminate the sublicense upon [\*\*] days written notice to AMT. For any sublicenses granted by AMT to Amgen under this Section 8.5.1.1 (Grant of License), AMT shall maintain the underlying Third Party Agreement in full force and effect and shall promptly notify Amgen of any notice of default or other circumstances related to the underlying Third Party Agreement that may reasonably affect the sublicense granted to Amgen.

- 8.5.1.2 No Other Rights. Amgen acknowledges that the rights and licenses granted under Section 8.5.1.1 are limited to the scope expressly granted. Accordingly, except for the rights expressly granted under Section 8.5.1.1, no right, title, or interest of any nature whatsoever is granted whether by implication, estoppel, reliance, or otherwise, by AMT to Amgen. All rights that are not specifically granted herein are reserved to AMT. Without limiting the foregoing, Amgen hereby acknowledges that AMT retains the right to Exploit, and authorize (by license or otherwise) Third Parties to Exploit, any product other than a GDNF Product even if such product is covered by a claim within the AMT Licensed Patent Rights.
- 8.5.1.3 Limited Exploitation Rights. In the event that Amgen requests a license from AMT in accordance with Section 8.5.1.1 (Grant of License), without limiting the provisions of Section 8.5.1.2 (No Other Rights), Amgen agrees (on behalf of itself and its Affiliates), and shall cause each of its Sublicensees to agree as a condition to the grant of a Sublicense, not to Exploit any AMT Licensed Know-How or AMT Licensed Patent Rights in any use outside the Licensed Field or for any products other than GDNF Products.
- 8.5.1.4 Cooperation. The Parties shall work together in good faith for a period of [\*\*] days to transfer to Amgen any GDNF Product developed by AMT prior to the date of such termination, including by transferring Regulatory Filings related to the program and manufacturing of GDNF Products.
- 8.5.1.5 Exclusivity. AMT hereby agrees that for a period of three (3) years thereafter (i) neither it nor any of its Affiliates shall itself Exploit any Gene Therapy product that delivers

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GDNF or the gene that encodes GDNF, or in either case any fragment of GDNF that has Functional Activity and (ii) neither it nor any of its Affiliates shall grant to any Third Party a license under the AMT Licensed Patent Rights or the AMT Licensed Know-How to Exploit any Gene Therapy product that delivers GDNF or the gene that encodes GDNF, or in either case any fragment of GDNF that has Functional Activity. Notwithstanding the foregoing, in the event that AMT or its Affiliate enters into a Subsequent Distracting Transaction during such three (3) year period, AMT shall either (x) Divest the Subsequent Distracting Program, (y) terminate the Subsequent Distracting Program, or (z) pay Amgen (a) milestones with respect to such Subsequent Distracting Product(s) pursuant to Article 4 (Fees and Revenue Sharing Payments) hereunder on a retroactive basis; and (b) Revenue Sharing Payments with respect to such Subsequent Distracting Product(s) pursuant to Article 4 (Fees and Revenue Sharing Payments) hereunder on a going forward basis from the date of the closing of the Subsequent Distracting Transaction (in each case treating such Subsequent Distracting Product as a GDNF Product) until the end of the Payment Term. If AMT elects to Divest the Subsequent Distracting Program, then it will use its reasonable efforts to Divest such Subsequent Distracting Program. In the event AMT elects to Divest the Subsequent Distracting Program and fails to complete such Divestiture within [\*\*] of the closing of the Subsequent Distracting Transaction, then AMT will be deemed to have elected to pay Amgen milestones and royalties as set forth in the preceding sentence. If AMT elects to terminate the Subsequent Distracting Program then it will terminate all activities of such program (other than as required by Law) within [\*\*] days after the closing of the Subsequent Distracting Transaction.

**SECTION 8.6** For Amgen's Breach or for AMT's breach under Section 8.3.2. In the event that AMT terminates the Agreement under Section 8.2.2 or Amgen terminates the Agreement under Section 8.3.2 then Amgen and its Affiliates shall have no right to any license under the AMT Licensed Patent Rights and AMT Licensed Know-How or any right or license to any Regulatory Filings related to the program and manufacturing of GDNF Products.

**SECTION 8.7** Surviving Obligations and Provisions. In addition to any provision of this Agreement that expressly survives the termination of this Agreement, the provisions of Sections 4.1 (Milestone Fees) (with respect to milestones reached prior to such termination), 4.2 (Revenue Sharing Payments) (with respect to sales made before such termination, or pursuant to Section 8.4.2 (GDNF Product in Stock and payments received by AMT from Sublicensees before such termination), 7.1 (Patent Prosecution), 8.4 (Effects of Termination of this Agreement), 8.5 (Reversion Rights), 8.6 (For Amgen's Breach or for AMT's breach under Section 8.3.2) and 8.7 (Surviving Obligations and Provisions) and Articles 1 (Definitions), 6 (Reports, Payments and Records) (with respect to periods with sales of GDNF Products made before such termination or payments received before such termination, or pursuant to Section 8.4.2 (GDNF Product in Stock)), and 9 (General) (excluding Section 9.7.1 (Representations, Warranties and Covenants)) shall survive the termination of this Agreement. Except those provisions of this Agreement that expressly survive the termination of this Agreement, all other provisions of this Agreement shall terminate and be of no further effect upon the termination of this Agreement for any reason.

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## **ARTICLE 9. GENERAL**

**SECTION 9.1** Termination of Original Agreement. Effective as of the Effective Date, Amgen and AMT hereby expressly terminate the Original Agreement including, except to the extent set forth in this Agreement, any terms which expressly or impliedly survive the termination of the Original. Each of Amgen and AMT on behalf of itself and its Affiliates and successors releases and discharges the other and its Affiliates and successors from all duties and obligations whatsoever in respect of the Original Agreement, provided that each of Amgen and AMT and their successors do not waive any rights, action, proceedings, claims, costs, awards and damages which it may have against the other or any of its Affiliates or successors and which have arisen or which may arise in relation to the Original Agreement. This Agreement and all Schedules attached to this Agreement constitute the entire agreement between the Parties as to the subject matter of such documents. All prior and contemporaneous negotiations, representations, warranties, agreements, statements, promises and understandings are superseded and merged into, extinguished by and completely expressed by such documents. No Party shall be bound by or charged with any written or oral agreements, representations, warranties, statements, promises or understandings not specifically set forth in such documents.

**SECTION 9.2** Addresses and Notices. All notices, requests, reports and other communications provided in this Agreement shall be in writing and shall be deemed to have been made or given when received, if delivered by hand or sent by e-mail, facsimile or overnight courier:

If to Amgen:

Amgen Inc.  
One Amgen Center Drive

If to AMT:

Amsterdam Molecular Therapeutics  
PO Box 22506  
1100 DA  
Amsterdam, Netherlands  
Fax: 31 20 566 9272  
Attention: For billing: Finance (Financef@amtbiopharma.com)  
For all other issues: The Chief Executive Officer

Such addresses may be altered by written notice so given.

**SECTION 9.3** Applicable Law. This Agreement and its effect are subject to and shall be construed and enforced in accordance with the law of the State of New York, without regard to its conflicts of laws, except as to any issue which depends upon the validity, scope or enforceability of any Licensed Patent Right, which issue shall be determined in accordance with the laws of the country in which such patent was issued. Each of the Parties hereby irrevocably and unconditionally consents to submit to the exclusive jurisdiction of the courts of the State of New York for any matter arising out of or relating to this Agreement and the transactions contemplated hereby, and agrees not to commence any litigation relating thereto except in such courts. Each of the Parties hereby irrevocably and unconditionally waives any objection to the laying of venue of any matter arising out of this Agreement or the transactions contemplated hereby in the courts of the State of New York and hereby further irrevocably and unconditionally waives and agrees not to plead or claim in any such court that any such matter brought in any such court has been brought in an inconvenient forum. The Parties agree that a final judgment in any such matter shall be conclusive and may be enforced in other jurisdictions by suits on the judgment or in any other manner provided by law. Any proceeding brought by either Party under this Agreement shall be exclusively conducted in the English language.

**SECTION 9.4** Performance by Affiliates. Either Party shall have the right to exercise its rights and perform its obligations hereunder through its Affiliates, provided it shall remain responsible for such performance.

**SECTION 9.5** Confidential Information.

9.5.1 Obligations and Restrictions. A Party receiving Confidential Information under this Agreement or the Original Agreement shall, subject to Section 8.5.1.1, until [\*\*] years following the termination of this Agreement, maintain such Confidential Information in confidence, and shall not disclose, divulge or otherwise communicate such Confidential Information to others, except to directors, officers, employees, consultants, subcontractors, Sublicensees and/or agents who are bound by like terms of confidentiality, nor use it for any purpose, except pursuant to, and in order to carry out, the terms and objectives of this Agreement. The receiving Party further agrees to exercise reasonable precautions to prevent and restrain the unauthorized disclosure of such Confidential Information by any of its directors, officers, employees, consultants, subcontractors, Sublicensees and agents. Additionally, the terms of this Agreement and the Original Agreement will be held in confidence by the Parties until [\*\*] years following the termination of this Agreement or the Original Agreement, except to the extent necessary in order to enforce its rights under this Agreement or as required by the rules of any securities exchange or automated quotation system or to the extent such terms are required to be disclosed to a governmental agency, or in response to involuntary compulsory process issued by a court, administrative agency or any governmental body having apparent jurisdiction provided the Party subject to such process first provides notice to the other Party hereto (to the extent practicable) and reasonably cooperates with efforts by the notified Party to secure confidential

protection of such terms. The obligations of the Parties under this Section 9.5.1 are subject to the provisions of Section 9.5.2 and 9.5.3.

9.5.2 Release from Restrictions. The provisions of Section 9.5.1 (Obligations and Restrictions) shall not apply to any Confidential Information that:

- (i) was generally available to the public or otherwise part of the public domain at the time of its disclosure to the receiving Party through no fault of the receiving Party;
- (ii) became generally available to the public or otherwise part of the public domain after its disclosure through no fault of the receiving Party;
- (iii) at the time of receipt by a Party was independently known to such Party without obligation of confidentiality from a source other than the other Party to this Agreement or anyone acting on behalf of any of them;
- (iv) has been made available to such Party without obligation of confidentiality by a Third Party having the lawful right to do so without breaching any such obligation of nonuse or confidentiality to any Party to this Agreement; or
- (v) was independently developed by the receiving Party (without reference to or use of Confidential Information of the disclosing Party).

9.5.3 Activities Related to GDNF Products. The restrictions set forth in this Section 9.5 (Confidential Information) shall not prevent (i) a Party from preparing, filing, prosecuting or maintaining a patent application or its resulting patents related to a GDNF Product in accordance with the terms of this Agreement, or (ii) AMT from disclosing Confidential Information to governmental agencies to the extent required or desirable to secure government approval for the development, manufacture or marketing of a GDNF Product, or (iii) AMT from conducting Exploitation with respect to GDNF Products as permitted by this Agreement (including the disclosure of Confidential Information to Sublicensees), or (iv) a Party from disclosing Confidential Information of the other Party or the terms of this Agreement to: (a) its actual or potential investments bankers and other advisers (including financial, accounting and legal); (b) actual and prospective lenders or funders for the purpose of obtaining financing for its business; and (c) to a bona fide potential acquirer or merger partner for the purposes of evaluating entering into a merger or acquisition, provided, however, any such disclosure shall be subject to the Third Party receiving such Confidential Information or the terms of this Agreement being obligated to

substantially the same extent as set forth in this Section 9.5 (Confidential Information) to hold in confidence and not make use thereof for any purpose other than those permitted by this Agreement.

**SECTION 9.6** Compliance With Law; Severability. Nothing in this Agreement shall be construed to require the commission of any act contrary to Law. If this Agreement conflicts with

any statute, law, ordinance or treaty concerning the legal right of the parties to contract, the latter shall prevail. In such event, the affected provisions of this Agreement shall be curtailed and limited only to the extent necessary to bring it within the applicable legal requirements and the validity, legality and enforceability of the remaining provisions of this Agreement shall not in any way be affected or impaired thereby.

**SECTION 9.7** Representations, Warranties and Covenants.

9.7.1 Representations and Warranties. Each Party hereby represents and warrants to the other Party, as of the Effective Date, as follows:

- (i) Such Party is a corporation duly organized, validly existing and in good standing under the laws of the state in which it is incorporated;
- (ii) Such Party (a) has the corporate power and authority and legal right to enter into this Agreement, to perform its obligations and to grant the licenses hereunder, and (b) has taken all necessary corporate action on its part to authorize the execution and delivery of this Agreement and the performance of its obligations hereunder;
- (iii) This Agreement has been duly executed and delivered on behalf of such Party and constitutes a legal and valid obligation binding upon such Party and enforceable against it in accordance with its terms;
- (iv) The execution, delivery and performance of this Agreement by such Party do not conflict with any agreement, instrument or understanding, oral or written, to which it is a party or by which it is bound, nor violate any applicable Law;
- (v) Such Party is aware of no action, suit, inquiry or investigation instituted by any Third Party that questions or threatens the validity of this Agreement;
- (vi) All necessary consents, approvals and authorizations of all governmental authorities and other persons required to be obtained by such Party in connection with this Agreement have been obtained; and
- (vii) No Joint Know-How (as such term is defined in the Original Agreement) was generated by the Parties in connection with their activities under the Original Agreement.

9.7.2 Disclaimer. EXCEPT AS EXPRESSLY SET FORTH IN SECTION 9.7 (REPRESENTATIONS, WARRANTIES AND COVENANTS), NEITHER PARTY MAKES ANY REPRESENTATIONS OR WARRANTIES, EXPRESS OR IMPLIED, OF ANY KIND, INCLUDING AS TO MERCHANTABILITY, FITNESS FOR A PARTICULAR USE OR NON-INFRINGEMENT. SPECIFICALLY, NEITHER PARTY WARRANTS THE VALIDITY OR ENFORCEABILITY OF

THE PATENT RIGHTS LICENSED HEREUNDER, AND MAKES NO REPRESENTATIONS WHATSOEVER WITH REGARD TO THE SCOPE OF THE PATENT RIGHTS LICENSED HEREUNDER, OR THAT THE PATENT RIGHTS LICENSED HEREUNDER MAY BE EXPLOITED WITHOUT INFRINGING OTHER PATENTS OR OTHER INTELLECTUAL PROPERTY RIGHTS OF THIRD PARTIES.

9.7.3 Covenants. Each Party hereby covenants that it shall not enter into any agreement that is in conflict with this Agreement. Further, AMT covenants it shall not knowingly use any employee or consultant who is or has been debarred by the FDA or other regulatory agency, or, to the best of AMT's knowledge, is or has been the subject of debarment proceedings by the FDA or other regulatory agency.

9.7.4 AMT Acknowledgement. AMT hereby acknowledges that: (i) Amgen has granted certain rights under the Licensed Know-How and Licensed Patent Rights to MedGenesis Therapeutix Inc. and Biovail Laboratories International SRL outside of the Licensed Field; (ii) Amgen has granted certain academic institutions the right under material transfer agreements to conduct research (including preclinical and non-clinical activities but excluding in vivo and clinical activities) with respect to GDNF; and (iii) Amgen has placed a clinical hold order on its Regulatory Filings for the GDNF Product.

**SECTION 9.8** Waiver. A Party's consent to or waiver, express or implied, of the other Party's breach of its obligations hereunder shall not be deemed to be or construed as a consent to or waiver of any other breach of the same or any other obligations of the other Party. A Party's failure to complain of any act, or failure to act, by the other Party, to declare the other Party in default, to insist upon the strict performance of any obligation or condition of this Agreement or to exercise any right or remedy consequent upon a breach thereof, no matter how long such failure continues, shall not constitute a waiver by such Party of its rights hereunder, of any such breach, or of any other obligation or condition. A Party's consent in any one instance shall not limit or waive the necessity to obtain such Party's consent in any future instance and in any event no consent or waiver shall be effective for any purpose hereunder unless such consent or waiver is in writing and signed by the Party granting such consent or waiver.

**SECTION 9.9** Indemnity.

9.9.1 By Amgen. Amgen agrees to defend AMT and its directors, officers, employees and agents (the "**AMT Indemnified Parties**") at Amgen's cost and expense, and will indemnify and hold AMT and the other AMT Indemnified Parties harmless from and against any claims, losses, costs, damages, fees or expenses (including legal fees and expenses) (collectively, "**Losses**") resulting from any Third Party claim (including product liability claims and any claims made by any licensors under any Third Party Agreements described in Section 8.5.1.1) arising out of or otherwise relating to (i) the gross negligence or willful misconduct of Amgen, or (ii) breach of this Agreement or

the representations and warranties made hereunder by Amgen or (iii) following the grant of the licenses to Amgen under Section 8.5, the Exploitation of any GDNF Product by or on behalf of Amgen or its Affiliates or Sublicensees; except, in each case, to the extent such Losses result from clause (i), (ii), or (iii) of Section 9.9.2 (By AMT). In the event of any such claim against the AMT Indemnified Parties by a Third Party, AMT shall promptly notify Amgen in writing of the claim (**provided, however**, that any failure or delay to notify shall not excuse any obligations of Amgen except to the extent Amgen is actually prejudiced thereby) and Amgen shall solely manage and control, at its sole expense, the defense of the claim and its settlement; **provided, however**, that Amgen shall not settle any such claim without the prior written consent of AMT if such settlement does not include a complete release from liability or if such settlement would involve undertaking an obligation (including the payment of money by an AMT Indemnified Party), would bind or impair an AMT Indemnified Party, or includes any admission of wrongdoing or that any intellectual property or proprietary right of AMT is invalid or unenforceable. The AMT Indemnified Parties shall cooperate with Amgen and may, at their option and expense, be represented in any such action or proceeding by counsel of their own choosing.

9.9.2 **By AMT.** AMT agrees to defend Amgen and its directors, officers, employees and agents (the “**Amgen Indemnified Parties**”) at AMT’s cost and expense, and will indemnify and hold Amgen and the other Amgen Indemnified Parties harmless from and against any Losses resulting from any Third Party claim (including product liability claims) arising out of or otherwise relating to (i) the gross negligence or willful misconduct of AMT, (ii) the Exploitation of any GDNF Product by or on behalf of AMT or its Affiliates, or (iii) breach of this Agreement or the representations and warranties made hereunder by AMT; except, in each case, to the extent such Losses result from clause (i) or (ii) of Section 9.9.1 (By Amgen). In the event of any such claim against the Amgen Indemnified Parties by a Third Party, Amgen shall promptly notify AMT in writing of the claim (**provided, however**, that any failure or delay to notify shall not excuse any obligation of AMT except to the extent AMT is actually prejudiced thereby) and AMT shall solely manage and control, at its sole expense, the defense of the claim and its settlement; **provided, however**, that AMT shall not settle any such claim without the prior written consent of Amgen if such settlement does not include a complete release from liability or if such settlement would involve undertaking an obligation (including the payment of money by an Amgen Indemnified Party), would bind or impair an Amgen Indemnified Party, or includes any admission of wrongdoing or that any intellectual property or proprietary right of Amgen is invalid or unenforceable. The Amgen Indemnified Parties shall cooperate with AMT and may, at their option and expense, be represented in any such action or proceeding by counsel of their own choosing.

9.9.3 **No Liability for Pre-Effective Date Losses.** Neither Party shall assume or be liable for any Losses (whether by way of indemnity or otherwise) resulting from or arising in connection with the activities of the other Party (or any agent, independent contractor

or Third Party engaged by such other Party) or relating to such other Party’s intellectual property rights on or prior to the Effective Date.

9.9.4 **Insurance.** AMT shall at its own expense procure and maintain during the term of this Agreement and for [\*\*] years thereafter, insurance policy/policies, including product liability insurance, adequate to cover its obligations hereunder and which are consistent with normal business practices of prudent pharmaceutical companies and with AAV gene therapy trials generally. AMT’s insurance shall not be construed to create a limit of AMT’s liability with respect to its indemnification obligations under this Article 12 (General). AMT’s insurance hereunder shall be primary with respect to the obligations for which it is liable hereunder.

**SECTION 9.10 LIMITATION OF DAMAGES.** IN NO EVENT SHALL EITHER PARTY BE LIABLE HEREUNDER TO THE OTHER PARTY FOR ANY INDIRECT, SPECIAL, INCIDENTAL EXEMPLARY OR CONSEQUENTIAL DAMAGES (INCLUDING LOST REVENUE, LOST PROFITS, OR LOST SAVINGS), EVEN IF IT HAS NOTICE OF THE POSSIBILITY OF SUCH DAMAGES. The limitations set forth in this Section 9.10 (Limitation of Damages) shall not apply with respect to (i) any breach of Section 9.5 (Confidential Information) or (ii) the willful misconduct of a Party. NOTHING IN THIS SECTION 9.10 (LIMITATION OF DAMAGES) IS INTENDED TO LIMIT OR RESTRICT THE INDEMNIFICATION RIGHTS OR OBLIGATIONS OF EITHER PARTY UNDER SECTION 9.9 (INDEMNITY) WITH RESPECT TO ANY DAMAGES PAID BY THE OTHER PARTY TO A THIRD PARTY IN CONNECTION WITH A THIRD PARTY CLAIM.

**SECTION 9.11** The limitations of liability and exclusion of damages herein, shall apply even if a Party had or should have had knowledge, actual or constructive, of the possibility of such damages. In addition, such limitations of liability and exclusion of damages, and the sole and exclusive remedies provided herein: (i) are fundamental elements of this Agreement, which would not be entered into without such limitations, exclusions and sole/exclusive remedies; (ii) shall apply whether a claim is based on breach of contract, breach of warranty, tort (excluding fraud or intentional misconduct) or otherwise; and (iii) shall apply even if the breach is a total and/or fundamental breach of this Agreement and regardless of whether the limited damages or remedies fail of their essential purpose and/or fail to provide relief to a Party.

**SECTION 9.12 Assignment.** Neither this Agreement nor any right or obligation hereunder may be assigned or otherwise transferred by a Party (whether voluntarily, by operation of law or otherwise), without the prior express written consent of the other Party; **provided, however**, that either Party may, without the other’s consent, assign this Agreement and its rights and obligations hereunder in connection with the transfer or sale of all or substantially all of its business related to this Agreement whether in a merger, consolidation, acquisition or similar transaction. Notwithstanding anything contained in the foregoing to the contrary, either Party may assign this Agreement to its Affiliates. Any permitted assignee shall assume all obligations of its assignor under this Agreement. Any purported assignment or transfer in violation of this Section 9.12 (Assignment) shall be void.

**SECTION 9.13 Non-Use of Names.** Amgen shall not use the name, trademark, logo, physical likeness or name of AMT or any of its officers, directors or employees, or any adaptation of any of them, in any advertising, promotional or sales literature, without AMT’s prior written consent. Amgen shall require its Affiliates and Sublicensees to comply with the foregoing. AMT shall not use the name, trademark, logo, physical likeness or name of Amgen or any of its officers, directors or employees, or any adaptation of any of them, in any advertising, promotional or sales literature, without Amgen’s prior written consent. AMT shall require its Affiliates and Sublicensees to comply with the foregoing.

**SECTION 9.14 Publicity.** Neither Party shall originate any publicity, news release or other public announcement, written or oral, of this Agreement or its terms, without the prior approval of the other Party except solely to the extent a Party reasonably believes same is otherwise required by Law. Any Press Release

in connection with the signing of this Agreement is set out in Schedule 9.14. Each Party shall to the extent consistent with applicable Laws and regulations limit the disclosure of the financial terms set forth in this Agreement (such as by requesting confidential treatment of such terms in documents required to be filed with the U.S. Securities and Exchange Commission). AMT shall provide a proposed draft to Amgen of any intended press releases or other public disclosure, which AMT is required to make under applicable Law or stock exchange rule to the extent relating to this Agreement or the activities contemplated under this Agreement as soon as practicable and when possible at least [\*\*] business days in advance of its proposed release. Amgen shall consider AMT's request to make such press release or statement and within [\*\*] business days of receiving such press release or statement or sooner if reasonably requested by AMT, indicate to AMT whether Amgen requires any changes to be made prior to the release thereof, which changes AMT shall be required to make prior to making such press release or other public disclosure, unless the making of such changes would, in AMT's reasonable belief, put AMT in violation of a legal requirement to disclose a material fact pursuant to applicable Law or stock exchange rules. Notwithstanding the foregoing, if applicable Law or stock exchange rules, in the opinion of counsel, require disclosure in a time frame that makes prior disclosure by AMT to Amgen in accordance with the foregoing procedures impractical under the circumstances, AMT may make such disclosure provided that it promptly provide such disclosure to Amgen.

**SECTION 9.15 Construction.** The Parties agree that each Party has reviewed this Agreement and that any rule of construction to the effect that ambiguities are to be resolved against the drafting Party shall not apply to the interpretation of this Agreement. As used herein, the terms "include", "includes" and "including" shall be deemed to be immediately followed by "without limitation".

**SECTION 9.16 Headings.** The headings for each Article and Section in this Agreement have been inserted for convenience of reference only and are not intended to limit or expand on the meaning of the language contained in the particular Article or Section. Unless otherwise specified, (i) references in this Agreement to any Article, Section, or Schedule shall mean references to such Article, Section, or Schedule of this Agreement, (ii) references in any Section to any clause are references to such clause of such Section, and (iii) references to any agreement, instrument or other document in this Agreement refer to such agreement, instrument or other document as originally

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executed or, if subsequently varied, replaced or supplemented from time to time, as so varied, replaced or supplemented and in effect at the relevant time of reference thereto.

**SECTION 9.17 Further Actions.** Each Party agrees to execute, acknowledge and deliver such further instruments and to do all such other acts as may be necessary or appropriate in order to carry out the purposes and intent of this Agreement.

**SECTION 9.18 Independent Contractors.** The relationship between AMT and Amgen created by this Agreement is solely that of independent contractors. This Agreement does not create any agency, distributorship, employee-employer, partnership, joint venture or similar business relationship between the Parties. Neither Party is a legal representative of the other Party, and neither Party can assume or create any obligation, representation, warranty or guarantee, express or implied, on behalf of the other Party for any purpose whatsoever. Each Party shall use its own discretion and shall have complete and authoritative control over its employees and the details of performing its obligations under this Agreement.

**SECTION 9.19 No Benefit of Third Parties.** The representations, warranties, covenants and agreements set forth in this Agreement are for the sole benefit of the Parties hereto and their successors and permitted assigns, and they shall not be construed as conferring any rights on any Third Parties (with the exception of AMT Indemnified Parties and Amgen Indemnified Parties under Sections 9.9.1 and 9.9.2, respectively).

**SECTION 9.20 Counterparts.** This Agreement may be executed in any number of counterparts, each of which need not contain the signature of more than one Party but all such counterparts taken together shall constitute one and the same agreement, and may be executed through the use of facsimiles.

**SECTION 9.21 License to Intellectual Property.** The Parties acknowledge that this Agreement constitutes a license to "intellectual property" as such term is used in §365(n) of the United States Bankruptcy Code ("**Bankruptcy Code**"). The Parties shall retain and may fully exercise all of their respective rights and elections under the Bankruptcy Code. Following the commencement of a bankruptcy proceeding of a Party, the non-bankrupt Party shall further be entitled to a complete duplicate of (or complete access to, as appropriate) any such intellectual property, and such, if not already in its possession, shall be promptly delivered to the non-bankrupt Party, unless the bankrupt Party elects to continue, and continues, to perform its obligations under this Agreement.

**SECTION 9.22 English Language.** This Agreement, including all Schedules, is being executed in the English language only, which language shall be controlling in all respects, and all versions hereof in any other language shall be for accommodation only and shall not be binding upon the Parties hereto. All communications to be made or given pursuant to this Agreement shall be made in the English language.

[The rest of this page left intentionally blank.]

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IN WITNESS WHEREOF, the Parties have each caused a duly authorized officer to sign this Agreement to be effective as of the Effective Date.

**AMGEN INC.**

By: /s/ Roger M. Perlmutter  
Name: Roger M. Perlmutter  
Title: EVP Research & Development

**AMSTERDAM MOLECULAR THERAPEUTICS**

By: /s/ P.J. Morgan CFO  
Name: Jörn Aldag  
Title: Chief Executive Officer

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## **SCHEDULE 1.10**

### **(AMT Licensed Patent Rights)**

Country	Official No.	Filing Date	Case Status
【**】	【**】	【**】	【**】

Confidential Materials omitted and filed separately with the Securities and Exchange Commission. A total of one page was omitted. 【\*\*】

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## **SCHEDULE 1.50**

### **(Licensed Patent Rights)**

#### GDNF Related IP

Country	Filing Number	Filing	Status
【**】	【**】	【**】	【**】

Confidential Materials omitted and filed separately with the Securities and Exchange Commission. A total of four pages were omitted. 【\*\*】

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## **SCHEDULE 9.14**

### **(Press Release)**

#### **Amsterdam Molecular Therapeutics Amends Amgen GDNF Gene License Agreement**

**Amsterdam, The Netherlands, DATE, 2010-Amsterdam Molecular Therapeutics (AMT) Holding N.V. (Euronext: AMT), a leader in the development of gene based therapies, today announced that it has amended and restated its licensing agreement with Amgen (Nasdaq: AMGN) for gene therapy applications incorporating the GDNF (glial cell derived neurotrophic factor) gene to which Amgen holds rights. Financial terms were not disclosed.**

The GDNF gene contains the information to produce a protein necessary for the development and survival of nerve cells. The positive effect of GDNF on nerve cells has already been demonstrated in early research. Studies with a GDNF gene therapy, AMT-090, in a Parkinson's disease model are being conducted by AMT in collaboration with the University of Lund, Sweden. AMT also plans to combine the GDNF gene with its proprietary adeno-associated virus (AAV) technology to develop gene therapies for a range of CNS applications, such as Huntington's disease and amyotrophic lateral sclerosis (ALS), with an aim to protect and enhance the function of the affected nerve cells.

"Based on the promising early results of our GDNF gene therapy product in Parkinson's disease models, we believe there is an opportunity for a similar approach in other debilitating CNS disorders. For many of these disorders, current therapies are limited and tend only to treat symptoms. Treatment with our gene therapies has the potential to halt or reverse disease progress," said Jurn Aldag, CEO of AMT, "This agreement will allow us to progress the program for Parkinson's Disease forward and at the same time find a partner who will support the funding of our GDNF programs in alternative indications. Expanding the partnering opportunities could mean even greater interest as the widened therapeutic applications offer more chances of success, potentially less complex product development paths and in many cases fewer patients to enroll in clinical trials."

#### **About Amsterdam Molecular Therapeutics**

AMT is a leader in the development of human gene based therapies. Using adeno-associated viral (AAV) derived vectors as the delivery vehicle of choice for therapeutic genes, the company has been able to design and validate what is probably the first stable and scalable AAV production platform. This proprietary platform can be applied to a large number of rare (orphan) diseases that are caused by one faulty gene. Currently, AMT has a product pipeline with several AAV-based gene therapy products in LPLD, Hemophilia B, Duchenne Muscular Dystrophy, Acute Intermittent Porphyria, and Parkinson's Disease at different stages of research or development. AMT was founded in 1998 and is based in Amsterdam.

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For further enquiries:

Jörn Aldag CEO AMT Tel: +31 20 566 7394 j.aldag@amtbiopharma.com	Mike Sinclair Partner Halsin Partners Tel: +44 20 7318 2955 msinclair@halsin.com
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*Certain statements in this press release are "forward-looking statements" including those that refer to management's plans and expectations for future operations, prospects and financial condition. Words such as "strategy," "expects," "plans," "anticipates," "believes," "will," "continues," "estimates," "intends," "projects," "goals," "targets" and other words of similar meaning are intended to identify such forward-looking statements. Such statements are based on the current expectations of the management of AMT only. Undue reliance should not be placed on these statements because, by their nature, they are subject to known and unknown risks and can be affected by factors that are beyond the control of AMT. Actual results could differ materially from current expectations due to a number of factors and uncertainties affecting AMT's business. AMT expressly disclaims any intent or obligation to update any forward-looking statements herein except as required by law.*



Confidential Materials omitted and filed separately with the  
Securities and Exchange Commission. Double asterisks denote omissions.

DATA LICENSE AGREEMENT

between

THE REGENTS OF THE UNIVERSITY OF CALIFORNIA

and

uniQure biopharma B.V.

for

AAV2.GDNF Data

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DATA LICENSE AGREEMENT

for

AAV2.GDNF Data

This license agreement (“Agreement”) is made effective this 12<sup>th</sup> day of June, 2012 (“Effective Date”), by and between The Regents of the University of California, a California corporation, having its statewide administrative offices at 1111 Franklin Street, 12th Floor, Oakland, California 94607-5200 (“The Regents”), acting through its Office of Technology Management, University of California, San Francisco, 185 Berry Street Suite 4603, San Francisco, California, 94107 (“UCSF”) and uniQure biopharma B.V. (uniQure) a Netherlands corporation, having a principal place of business at Meibergdreef 61, 1105 BA Amsterdam, the Netherlands (“Licensee”). The Regents and Licensee are sometimes referred to herein individually as a “Party” and collectively as the “Parties”.

## BACKGROUND

- A. Licensee is a company engaged in the research, development, manufacturing and commercialization of gene therapy products, including research and development of therapeutics for the treatment of neurological diseases and other diseases.
- B. Certain technical information relating to pre-clinical work on GDNF.AAV2, including the preparation of the IND (as defined in the Collaboration Agreement, defined below) (such information being included in the definition of Data set out in the Collaboration Agreement), was made in the course of research at UCSF by [\*\*].
- C. The development of such Data was sponsored in part by National Institutes of Health and, as a consequence, this license may be subject to overriding obligations to the United States Federal Government under 35 U.S.C. §§ 200-212 and applicable regulations.

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- D. The Licensee and The Regents intend to collaborate on the development of GDNF.AAV2 product for the treatment of Parkinson’s Disease in accordance with the terms of the preceding Collaboration Agreement (as defined below) and further Data may be developed during the collaboration as described in Article 1 of the Collaboration Agreement.
- E. The Licensee wishes to obtain certain rights in respect of the Data from The Regents for the commercial development of a GDNF.AAV2 gene therapy product for the treatment of Parkinson’s Disease, in accordance with the terms and conditions set forth herein and The Regents are willing to grant those rights so that the Products may be developed and the benefits enjoyed by the general public.
- F. The Licensee acknowledges that: (i) consideration for Data is due to early access; and (ii) some of the Data may become public in accordance with the NINDS Agreement (defined in the Collaboration Agreement) without a decrease in consideration due to The Regents under this Agreement.

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The Parties agree as follows:

### 1. DEFINITIONS

The definitions defined in the Collaboration Agreement shall also apply to this Agreement. As used in this Agreement, the following additional terms, whether used in the singular or plural, shall have the following meanings:

- 1.1 “AAV” means recombinant adeno-associated virus.
- 1.2 “Affiliate” means any Person which controls, is controlled by or is under common control with a Party. For purposes of this Section 1.2 (“Affiliate”), “control” shall mean (i) in the case of corporate entities, direct or indirect ownership of fifty percent (50%) or more of the stock or

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shares entitled to vote for the election of directors and (ii) in the case of non-corporate entities, direct or indirect ownership of fifty percent (50%) or more of the equity or income interest therein. Notwithstanding the preceding provisions, with respect to an Affiliate of a Party to this Agreement, once such entity ceases to be an Affiliate of such Party, then, without any further action, such entity shall cease to have any rights, including license and sublicense rights, under this Agreement by reason of being an Affiliate.

- 1.3 “Bundle” means an Identified Product (a) Sold together with another pharmaceutical product for a single price including fixed combinations or (b) Sold as part of a delivery agent (such as a viral vector) which contains two or more different therapeutic genes or therapeutic fragments of genes and not all of the therapeutic genes or fragments of genes encode GDNF or a fragment of GDNF that has Functional Activity. For clarity, the other pharmaceutical product or other element contained in a delivery agent shall not include excipients, buffers or other similar substances that are typically formulated with the drug products contained in the Identified Product to form the final Identified Product for Sale, nor standard, off-the-shelf delivery devices such as syringes, but may include specialized drug delivery devices, other active drug substances or other proprietary materials intended to deliver the drug contained in the Bundle.
- 1.4 “Calendar Quarter” means each respective period of three (3) consecutive months ending on March 31, June 30, September 30 and December 31 of each Calendar Year.
- 1.5 “Calendar Year” means each respective period of twelve (12) months commencing on January 1 and ending on December 31.
- 1.6 “Collaboration Agreement” means the collaboration agreement between The Regents and the Licensee dated the same date as this Agreement.

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- 1.7 “Commercially Reasonable Efforts” means, with respect to a particular Identified Product and those diligent activities hereunder with respect to such Identified Product including diligent development and commercialization, those efforts and resources that Licensee would normally use with respect to a gene therapy product, which product is owned by Licensee, or to which Licensee has similar rights, with a similar market potential at a similar stage in development or product life as the Identified Product, taking into account all relevant factors, based on the facts and circumstances at the time, including without limitation diligent

progress, safety and efficacy issues, other medical and clinical considerations, product labeling or anticipated labeling, product profile, present and future market potential, past performance of similar products (including both Identified Products and Licensee's own pharmaceutical products that are of similar market potential), competitive market conditions, the likely timing of the product's entry into the market, financial considerations, intellectual property considerations and the likelihood of regulatory approval.

1.8 "Earned Royalty" has the meaning set out in Section 5.1.

1.9 "Exploit" means to research, have researched, develop, have developed, make, have made, use, have used, offer for Sale, have offered for Sale, Sell, have Sold, import, have imported, export, have exported or otherwise exploit, or transfer possession of or title in, a Product. Cognates of the word "Exploit" shall have correlative meanings.

1.10 "Field" means the use of Data to Exploit Products for the therapeutic, palliative and prophylactic treatment of Parkinson's Disease in humans. The Field specifically excludes all other uses and applications. For the avoidance of doubt any item of Data shall not be deemed to have been used in relation to any other use or application unless it either (a) does not become published, publically available information, through no breach of this Agreement by either Party,

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in a format that can be used by another entity, in a similar manner in which Licensee can use Data, for any clinical trial or regulatory filings, (b) is material or (c) has been specifically and identifiably incorporated in, and relied on in any regulatory filing.

1.11 "Functional Activity" means neurotrophic activity with respect to dopaminergic neurons with an ability to (i) signal through the GDNF receptor complex and (ii) (a) increase tyrosine hydroxylase immunoreactivity or (b) protect against neurotoxin-mediated cell death.

1.12 "GDNF" means glial-cell derived neurotrophic factor.

1.13 "Identified Product" means a Product the identification or development of which materially uses the Data.

1.14 "Launch" means the date of the first commercial Sale by Licensee of an Identified Product.

1.15 "Net Revenue" means the total gross proceeds (including, without limitation, any license fees, maintenance fees, royalties or milestone payments), whether consisting of cash or any other forms of consideration received or collected by the Licensee from any Third Party Licensee in consideration of the grant of a Third Party License. Notwithstanding the foregoing, Net Revenue shall not include proceeds attributed in such Third Party License to bona fide (i) debt financing; (ii) equity (and conditional equity, such as warrants, convertible debt and the like) investments in the Licensee; and (iii) reimbursement for the cost of research and/or development services specifically dedicated to the development of Identified Products to be provided on a going forward basis by Licensee for the applicable Third Party Licensee under such Third Party License on the basis of Licensee's external and internal costs and/or full-time equivalent ("FTE") efforts of personnel at or below commercially reasonable and standard FTE rates. For the

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avoidance of doubt, any gross proceeds meeting the definition set forth above in this Section 1.15 shall be "Net Revenue" irrespective of whether such gross proceeds are received under one or more separate agreements and irrespective of how such gross proceeds are referred to or characterized by the Licensee or the Third Party Licensee.

1.16 "Net Sale" means the gross invoiced Sales prices charged for Identified Products Sold by Licensee in arms length transactions to third parties (but not including Sales relating to transactions between Licensee, its Affiliates, and/or their respective agents ("Relationship-Influenced Sale"), unless for end use consumption), less the total of the following charges or expenses as determined in accordance with International Financial Reporting Standards (IFRS): (i) trade, cash, prompt payment and/or quantity discounts including promotional or service discounts; (ii) returns, allowances, rebates, chargebacks, or payments to government agencies; (iii) retroactive price reductions applicable to Sales of such product; (iv) only those normal and customary discounts and rebates or wholesaler's discounts and rebates given as part of a formulary program that are paid or credited to customers, third-party payers, health care systems, or administrators for an Identified Product that is included in such formulary program, as permitted by applicable law; (v) credits or allowances for product replacement, whether cash or trade; (vi) non-recoverable Sales taxes, excise taxes, tariffs and duties; (vii) bad debt, to the extent in which bad debt is defined as bad debt in Licensee's records and actually not collected; and (viii) freight or other transportation charges, insurance charges, additional special packaging, and other governmental charges. Notwithstanding the foregoing, the Parties intend that Net Sales exclude amounts invoiced for or revenues attributable to services ancillary to the delivery or use of Identified Products including surgical procedures, hospital stays or the like, as well as

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amounts invoiced for devices Sold in connection with any Identified Product as long as such amounts are separately identified in an invoice.

1.16.1 Any disposal of Identified Products at no charge for, or use of Identified Products at no charge in clinical or pre-clinical trials, and Identified Products given as free samples, or distributed at no charge to patients unable to purchase Identified Product shall not be included in Net Sales.

1.16.2 Upon any Sale or other disposal of any Identified Product for any consideration other than an exclusively monetary consideration on bona fide arm's length terms, then for purposes of calculating the Net Sales under this Agreement, such Identified Product shall be deemed to be Sold at the average Sales price for such Identified Product having the same dosage form and strength during the applicable reporting period in the country where such Sale or other disposal occurred when such Identified Product is Sold alone and not with other products, and if such Identified Product is not Sold alone in such country during the applicable reporting period, then such Identified Product shall be deemed to be Sold at the average Sales price during the applicable reporting period generally achieved for such Identified Product having the same dosage form and strength in the rest of the world.

1.16.3 For any Relationship-Influenced Sale of a Licensed Product, Net Sales shall be based on the gross invoiced Sales prices at which the Relationship-Influenced Sale Purchaser resells such Identified Product.

1.16.4 Where a Identified Product is Sold in a Bundle, then for the purposes of calculating the Net Sales under this Agreement, such Identified Product shall be deemed to be Sold for an amount equal to  $(X/(X+Y)) \times 2$ , where: X is the average Sales price during the applicable reporting period for such Identified Product being Sold alone (in the same dosage

form) (or, should more than one Identified Product be included in a Bundle with a product other than a Identified Product, the sum of such average Sales prices for the included Identified Products) in the particular country of Sale; Y is the sum of the average Sales price during the applicable reporting period in the particular country of Sale, when Sold alone, of each pharmaceutical (other than the included Identified Product(s)) included in the Bundle (in the same dosage form); and Z equals the Net Sales of such Bundle. In the event that a Identified Product or one or more of the other pharmaceuticals in the Bundle are not Sold separately (in the same dosage form), the Parties will discuss in good faith to determine an equitable fair market price to apply to such Identified Product or other pharmaceutical in the Bundle.

1.17 “Payment Term” means, with respect to a given country, the period of time commencing on Launch of the first Identified Product in such country and continuing until the tenth (10th) anniversary of the Launch of such Identified Product in such country.

1.18 “Person” means an individual, corporation, partnership, limited liability company, trust, business trust, association, joint stock company, joint venture, pool, syndicate, sole proprietorship, unincorporated organization, governmental authority or any other form of entity not specifically listed herein.

1.19 “Product” means any pharmaceutical product which contains or consists of an AAV2 genetic construct encoding GDNF or any fragment of GDNF that has Functional Activity.

1.20 “Relationship-Influenced Sale Purchaser” means the purchaser of Identified Product in a Relationship-Influenced Sale.

1.21 “Sale” means the act of selling, leasing or otherwise transferring, providing, or furnishing for use for any consideration. Correspondingly, “Sell” means to make or cause to be made a Sale and “Sold” means to have made or caused to be made a Sale.

1.22 “Third Party License” means a grant of any right or license by Licensee in respect of the Data to Exploit Identified Products.

1.23 “Third Party License Fees” is defined in Section 4.1.

## 2. GRANT

2.1 Subject to the limitations and other terms and conditions set forth in this Agreement, The Regents hereby grants to the Licensee a non-exclusive (subject to Section 2.3), royalty bearing, sublicensable (as described below) license under its rights in and to the Data, in the United States and in other countries throughout the world where The Regents may lawfully grant such licenses, only in the Field. Such license includes all activities to be carried out by the Licensee under the Collaboration Agreement.

2.2 As long as this Agreement is effective, The Regents agrees not to license the rights in and to Data granted to the Licensee in Section 2.1 to other for-profit entities.

2.3 The Regents reserves and retains the right (and the rights granted to the Licensee in this Agreement shall be limited accordingly) to make and use the Data and to make and use any Products and to practice any process that is the subject of the Data (and to grant any of the foregoing rights to other educational and non-profit institutions) for educational and research purposes, and including publication and other communication of any research results, provided, however, that The Regents shall not enter into sponsored research agreements for or on behalf of for-profit entities with:

(i) [\*\*]; and / or

(ii) the following members of [\*\*] research team (whilst they remain members of his research team): [\*\*],

in respect of any GDNF.AAV2 gene therapy product encoding GDNF (or any fragment of GDNF that has Functional Activity) for the treatment or prophylaxis of Parkinson’s Disease while the Data License and/or any Third Party License(s) is in effect, *provided, however*, the sponsored research restriction in this Section 2.3 shall expire upon the earlier of (i) the [\*\*] year anniversary after a Launch in the US, France, Germany, Italy, Spain or UK; or (ii) termination of this Agreement and any Third Party License(s).

2.4 The Regents also grants to the Licensee the right to sublicense the license granted according to Section 2.1 to its Affiliates and to Third Parties (who shall also have the right to sublicense as shall such sub-sub-licensees onwards through multiple tiers) (each such party receiving such a license (a Third Party License) being referred to as a Third Party Licensee) provided that the Licensee is responsible for making sure that: (a) any such Third Party License must include all of the rights of and obligations due to The Regents (and if applicable, the United States government) contained in this Agreement and (b) The Licensee shall notify The Regents of each Third Party License granted within [\*\*] days of its grant. Upon termination of this Agreement for any reason, all Third Party Licenses granted by Licensee shall survive such termination and The Regents shall assume all such Third Party Licenses as the licensor thereunder in accordance with the terms of such Third Party Licenses; provided, however, that (a) the Third Party Licensee is not in material default and agrees in writing to an assignment to The Regents of such sublicense, (b) all of the terms of this Agreement are agreed to fully in writing by such Third Party Licensee; and (c) the Third Party Licensee acquires no rights from or

obligations on the part of The Regents other than those that are specifically granted under this Agreement and assumes all liability and obligations to The Regents required of Licensee by this Agreement with respect to The Regents' sublicensed rights, including past due obligations existing at the time of assignment of this Agreement by Licensee. If any Third Party Licensee fails to meet the above provisions described in this Section 2.4 then The Regents may terminate its sublicense, in accordance with Article 11 (Termination by The Regents). The Regents will not be bound to perform any duties or obligations set forth in any sublicense to any Third Party Licensee that extend beyond the duties and obligations of The Regents set forth in this Agreement, and the Licensee's obligations to The Regents hereunder will be binding upon each Third Party Licensee. Any such assignment will include a modification to the sublicense that requires payment of Earned Royalties directly to The Regents by the Third Party Licensee as if it were the Licensee at the rate set forth in Article 5 (Earned Royalties) and payment of any Third Party License Fees as set forth in Article 4 (Payments In Respect Of Third Party Licenses) in accordance with Article 6 (Payment Terms).

### 3. IND APPROVAL PAYMENT

3.1 In consideration of the approval of the IND, the Licensee will pay to The Regents three hundred thousand U.S. dollars (\$300,000) within [\*\*] days of the Effective Date and provided that the Licensee has received a proper invoice from The Regents for such sum. This payment is non refundable, non-cancelable and is not an advance or otherwise creditable against any royalties or other payments required to be paid under the terms of this Agreement.

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### 4. PAYMENTS IN RESPECT OF THIRD PARTY LICENSES

4.1 During the period from the Effective Date to the end of the Payment Term on a country by country basis, the Licensee will pay to The Regents the following third party license fees ("Third Party License Fees"):

4.1.1 [\*\*] percent ([\*\*]%) of all Net Revenue received by Licensee from any Third Party Licensee if the Third Party License between the Licensee and such Third Party Licensee is executed [\*\*];

4.1.2 [\*\*] percent ([\*\*]%) of all Net Revenue received by Licensee from any Third Party Licensee if the Third Party License between the Licensee and such Third Party Licensee is executed [\*\*];

4.1.3 [\*\*] percent ([\*\*]%) of all Net Revenue received by Licensee from any Third Party Licensee if the Third Party License between the Licensee and such Third Party Licensee is executed [\*\*];

4.1.4 [\*\*] percent ([\*\*]%) of all Net Revenue received by Licensee from any Third Party Licensee if the Third Party License between the Licensee and such Third Party Licensee is executed [\*\*]; and

4.1.5 [\*\*] percent ([\*\*]%) of all Net Revenue received by Licensee from any Third Party Licensee if the Third Party License between the Licensee and such Third Party Licensee is executed [\*\*].

4.2 For the avoidance of doubt, each of the Third Party License Fees will be payable with respect to each Identified Product.

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4.3 Should the Licensee be required to pay a license payment from Net Revenue to an unaffiliated third party(ies) for material intellectual property rights needed in order to make, use, Sell or import Identified Products, then Licensee may reduce the payment from Third Party License Fees due The Regents as follows. If Licensee is required to pay The Regents and such unaffiliated third party(ies) from the same form of payment (e.g. license fee, maintenance fee, royalty or milestone payment) under Net Revenue, then the Third Party License Fee to be paid to The Regents by Licensee shall be reduced by the lesser of either (i) an amount equal to the applicable license payment due to the unaffiliated third party(ies), or (ii) the amount up to [\*\*] percent ([\*\*]%) of the original Third Party License Fee amount due The Regents. For the avoidance of doubt, in no event shall the amount paid to The Regents be reduced below [\*\*] percent ([\*\*]%) of the original Third Party License Fee amounts due The Regents. For example purposes only, if Licensee is required to pay The Regents and an unaffiliated third party royalty payments from Net Revenue, then the reduction under this Section 4.3 shall be applied only to such royalty payment and further, such reduction shall not be applied to any other form of payment due to The Regents such as milestone payments received from any third party.

4.4 All Third Party License Fees are non-cancelable and are not an advance or otherwise creditable against any royalties or other payments required to be paid under the terms of this Agreement.

### 5. EARNED ROYALTIES

5.1 During the Payment Term, the Licensee will also pay to The Regents a royalty of [\*\*] percent ([\*\*]%) of the Net Sales of Identified Products Sold by the Licensee ("Earned Royalty"). For the avoidance of doubt, Earned Royalties shall not be payable in respect of Sales made by Third Party Licensees, but for which The Regents will receive payment on royalties with respect

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to Sales made by Third Party Licensees as part of the Third Party License Fees according to Article 4.

5.2 In the event it becomes necessary for Licensee to license intellectual property rights from an unaffiliated third party, and Licensee is required to pay a royalty percentage of the Net Sales of Identified Products Sold by the Licensee to that unaffiliated third party in order to make, use, Sell or import Identified Products, and the combined earned royalties due The Regents and unaffiliated third parties exceeds [\*\*] percent ([\*\*]%), then the Earned Royalty to be paid to The Regents by Licensee shall be reduced by an amount equal to [\*\*] the excess over [\*\*]percent ([\*\*]%) of the royalty rate(s) due to the unaffiliated third party(ies). However, in no event shall the amount paid to The Regents be reduced below [\*\*] percent ([\*\*]%) of the original Earned Royalty amounts due The Regents.

6. PAYMENT TERMS

6.1 Prior to the first Launch of an Identified Product, Licensee shall pay The Regents any Third Party License Fees within [\*\*] days that the Licensee receives any Net Revenue in respect of which such Third Party License Fees are payable.

6.2 After the first Launch of an Identified Product and during the Payment Term, Licensee will prepare and deliver reports to The Regents detailing all Earned Royalties and Third Party License Fees for each Calendar Quarter within [\*\*] days of the end of each such Calendar Quarter and such reports will be accompanied by payment of such Earned Royalties and Third Party License Fees. The reports will show: the gross Sales and Net Sales of Identified Products Sold by Licensee during the most recently completed Calendar Quarter; the number of each type of Identified Products Sold by Licensee; Earned Royalties, in U.S. dollars, due on Net Sales; and the exchange rates used, if any, together with all Third Party License Fees.

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6.3 Invoices. To the extent an invoice is required to be submitted by The Regents to Licensee, such invoice shall be addressed to:

uniQure biopharma B.V.  
FAO Finance Department  
P.O.Box 22506  
1100 DA Amsterdam  
The Netherlands  
Email: Finance@uniQure.com

Invoices not submitted to the foregoing address may be subject to delay or return.

6.4 All consideration due to The Regents will be payable and will be made in United States dollars by check payable to "The Regents of the University of California" or by wire transfer to an account designated by The Regents. The Licensee is responsible for all bank or other transfer charges save for any charges levied by The Regents' bank which shall be the responsibility of The Regents. When Identified Products are Sold for, or Third Party License Fees are received in, monies other than United States Dollars (each a "Foreign Currency Amount"), the Earned Royalties and Third Party License Fees will be converted into equivalent United States Dollars. The exchange rate will be that which corresponds to the rate used by Licensee, for the respective reporting period, related to recording such Foreign Currency Amount in its books and records that are maintained in accordance with IFRS, provided, however, that if, at such time, Licensee does not use a rate for converting into a U.S. dollar equivalent that is maintained in accordance with IFRS, then Licensee will use a rate of exchange which corresponds to the rate of exchange for such currency into U.S. dollars quoted in The Wall Street Journal Internet U.S. Edition, as of the last day of the applicable reporting period (or, if unavailable on such date, the first date thereafter on which such rate is available).

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6.5 Payment of Taxes. All excises, taxes, and duties, excluding any value-added tax (collectively "Taxes"), only to the extent actually incurred and not reimbursable, refundable or creditable under the tax authority of any country, levied on account of a payment made by Licensee to The Regents pursuant to this Agreement will be the responsibility of and paid by The Regents or shall be subject to the withholding, remittance, and offset provisions of Section 6.6.

6.6 Withholding by Licensee. In the event that law or regulation requires Licensee to withhold Taxes with respect to any payment to be made by Licensee pursuant to this Agreement, Licensee will withhold such Taxes from the amount due and furnish, within [\*\*] days of the date of such withholding, The Regents with proof of payment of such Taxes. Licensee will provide reasonable assistance to The Regents in its efforts to claim an exemption of Taxes, obtain a refund of Taxes withheld, or obtain a credit with respect to such Taxes paid. In order for The Regents to secure an exemption from, or a reduction in, any withholding of Taxes, The Regents shall provide to Licensee such forms as reasonably required for each type of payment to be made pursuant to the Agreement for which an exemption from, or a reduction in, any withholding of Taxes is claimed. In the event that a form previously furnished to Licensee expires, is incorrect, or is inapplicable to the type of payment to be made, due to a change in circumstances or otherwise, The Regents shall furnish a new form to Licensee prior to the payment of any such amount in order to secure an exemption from, or a reduction in, any withholding of Taxes with respect to such payment.

6.7 In the event that royalties, fees, or other monies owed to The Regents are not received by The Regents when due, the Licensee will pay to The Regents interest at a rate of [\*\*] percent ([\*\*]%) simple interest per annum. Such interest will be calculated from the date payment was

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due until actually received by The Regents. Such accrual of interest will be in addition to and not in lieu of, enforcement of any other rights of The Regents due to such late payment.

7. BOOKS AND RECORDS

7.1 Licensee shall keep true and fair books and records showing all Identified Products Sold under this Agreement (for the purposes of showing the amount of earned Royalties payable to The Regents). Licensee shall preserve these books and records for at least [\*\*] years from the date of the Earned Royalty payment to which they relate. The Regents shall have the right, at its own expense and not more than [\*\*] during the term of this Agreement, to have an independent, certified public accountant, selected by The Regents, audit the records of Licensee in the location(s) where such records are maintained upon reasonable notice (which shall be no less than [\*\*] days prior written notice) and during regular business hours, and for the sole purpose of verifying the basis and accuracy of payments required and made under this Agreement. The books and records for any particular Calendar Year shall only be subject to [\*\*]. The report and communication of such accountant with respect to such an audit shall be limited to a certificate stating whether any report made or payment submitted by Licensee during such audited period is accurate or inaccurate and the amount of any payment discrepancy. Such accountant shall provide The Regents and Licensee with a copy of each such report simultaneously. The Regents shall bear the costs of any examination of books and records, except that if an error in royalties of more than [\*\*] percent ([\*\*]%) of the total Earned Royalties due for any year is discovered, Licensee shall bear the cost of that examination. Upon the expiration of [\*\*] years following the end of any Calendar Year, the right to audit the books and records for such Calendar Year shall expire and the calculation of payments payable with respect to each such Calendar Year shall be binding and conclusive upon The Regents, and Licensee shall be released from any liability or



accountability with respect to payments for such Calendar Year. Licensee shall no longer be required to retain such records for such Calendar Year after the expiration of such [\*\*] year period. The Regents shall, and shall ensure that the independent accountant shall, treat all financial information subject to review under this Section 7.1 as Confidential Information of Licensee.

## 8. DILIGENCE

8.1 The Regents shall have the right to terminate this Agreement in its entirety if, by 31 December 2012, the Licensee, including affiliated companies, has not received at least €[\*\*] Euros) during the course of the calendar year of 2012 by way of equity and/or debt financings and/or payments by third party pharma/life sciences companies *provided, however*, that (a) the Parties shall meet within the [\*\*] day period after receipt of a Notice of Default (as defined below in Section 11.1) to agree in good faith whether the Licensee has nevertheless raised sufficient funds to be able to comply with its obligations under this Agreement and the Collaboration Agreement and (b) the termination shall not become effective at the end of the [\*\*] day Notice Period (defined below in Section 11.2) if either (i) the parties agree that the Licensee has raised sufficient funds to be able to comply with its obligations under this Agreement and the Collaboration Agreement; or (ii) the Licensee receives additional funds during such [\*\*] day Notice Period sufficient to reach the €[\*\*] Euros) threshold for the [\*\*]-period starting from 1 January 2012, as shown by written tangible evidence; or (iii) if the Licensee provides The Regents, within [\*\*] days of receipt of The Regents' Notice of Default, with a plan for raising such funds ("Funding Plan") within a specified time period, and The Regents accepts such Funding Plan, and Licensee thereafter continuously continues such implementation until such funds are received within the specified time period. The Regents will

notify Licensee of either The Regents' acceptance or rejection, in its sole discretion, of Licensee's Funding Plan. Any decision by The Regents to reject a Funding Plan pursuant to this Section 8.1 will be deemed final and The Regents shall have the right to immediately terminate this Agreement.

8.2 The Licensee will complete the following tasks, within [\*\*] months of the Effective Date of this Agreement:

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8.3 The Licensee, from the date it receives the Interim Report, agrees to use Commercially Reasonable Efforts to proceed, either directly or through a Third Party Licensee, to develop at least one Identified Product, and seek to obtain regulatory approval for such Identified Product in the U.S. and the European Union, and to use Commercially Reasonable Efforts to market the same in quantities sufficient to meet the market demands therefor in the U.S. and in France, Germany, Italy, Spain and the UK.

8.4 The Parties acknowledge that developing and seeking regulatory approvals for an Identified Product may include sequential implementation of clinical trials and intervals between clinical trials for data interpretation and clinical program planning and approval. The Regents acknowledge that in determining whether Licensee has met its requirement to use Commercially Reasonable Efforts in accordance with Section 8.3, reference will be made to the totality of Licensee's activities with respect to all Identified Product(s) (if, in Licensee's sole discretion, it decides to develop more than one Identified Product) in all countries, not on a country-by-country basis.

8.5 The Licensee will use Commercially Reasonable Efforts to obtain all necessary regulatory approvals in each country where Identified Products are manufactured, used, Sold, offered for Sale or imported.

8.6 Subject to Section 8.7, if the Licensee materially fails to comply with any of the provisions of Sections 8.1 to 8.5, then The Regents has the right and option to either terminate this Agreement or remove the licensing restriction in Section 2.2. This right, if exercised by The Regents, supersedes the rights granted in Article 2 (Grant) and The Regents shall be free to license the rights granted in Section 2.1 to other for-profit entities.

8.7 To exercise either the right to terminate this Agreement or to remove the licensing restriction in Section 2.2 for the Licensee's material failure to comply with any of the provisions of Sections 8.1 to 8.5, The Regents will give the Licensee written notice of the deficiency in accordance with Section 11.2 (Nonmonetary Default). If, after the Notice Period, or Notice Extension Period, if applicable, the deficiency has not been cured, then The Regents may, at its option, terminate this Agreement immediately or remove the licensing restriction in Section 2.2 by providing written notice to the Licensee.

## 9. PROGRESS REPORTS

9.1 Beginning on the date that is [\*\*] months after receipt by Licensee of the Interim Report, and continuing [\*\*] until the First Commercial Sale of an Identified Product, Licensee shall submit to The Regents a progress report covering Licensee's activities related to the development and testing of Identified Products. Progress reports must include a detailed summary of the following topics, but are not limited to: a summary of work completed, a current schedule of anticipated events or milestones, including the performance milestones outlined in Section 8 (Diligence); and, beginning [\*\*] months before the anticipated Launch of any

Identified Product, a detailed outline of the commercialisation plans for such Identified Product. If the Identified Product is being developed and commercialized by a Third Party Licensee, in whole or in part, the Licensee will summarize and deliver all reports due to The Regents from such Third Party Licensee.

9.2 The Licensee will submit to The Regents on the [\*\*] of the Effective Date of this Agreement a specific plan to develop and commercialize Identified Products "Commercial Development Plan". Such Commercial Development Plan shall include the timeline for developing Identified Products, including plans for preclinical studies, key aspects of the clinical development of Identified Products through regulatory approval, estimated dates for initiation and completion of clinical trials, manufacturing, sublicensing, and marketing and Sales. If actual progress differs from that anticipated in the Commercial Development Plan,

Licensee shall provide The Regents a written explanation for the reasons for the difference and a modified Commercial Development Plan within [\*\*] days of when Licensee is notified of such difference in progress.

## 10. TERM OF THE AGREEMENT

10.1 This Agreement shall commence on the Effective Date, and unless terminated earlier as provided in Articles 11 or 12, shall continue in full force and effect on a country-by-country basis until the end of the Payment Term with respect to such country and this Agreement will expire when all such payment obligations have ended in all countries (the **“Term”**). Upon expiration (but not an earlier termination) of the Payment Term in respect of a particular country, Licensee shall have a perpetual, non-exclusive, fully paid-up, royalty free license which includes the right to sublicense through multiple tiers of sublicense, under the Data to Exploit Identified Products in the Field in such country.

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## 11. TERMINATION BY THE REGENTS

11.1 **Monetary Default.** Upon any failure by Licensee to timely pay undisputed amounts due to The Regents required to be paid under this Agreement, The Regents, in addition to any other remedy available at law or equity, shall have the right to terminate this Agreement by giving Licensee written notice of such default (“Notice of Default”) in which Licensee has [\*\*] days’ from the effective date of such notice to repair such default. The Regents shall have the right to immediately terminate this Agreement upon the expiration of such [\*\*] day period by providing a written notice of termination (“Notice of Termination”) to Licensee, unless on or before such date Licensee has paid such amounts, in which case the Agreement shall continue unaffected. For the avoidance of doubt, disputed amounts shall be those on amounts that have been actually disputed by either Party pursuant to any audit conducted in accordance with Section 7 (Books and Records).

11.2 **Nonmonetary Default.** Upon any material non-monetary breach of this Agreement by Licensee (*i.e.*, not involving the payment by Licensee of any amounts required to be paid under this Agreement), The Regents, in addition to any other remedy available at law or equity, shall have the right to provide a Notice of Default to Licensee of such material non-monetary breach. In the event such material non-monetary breach has not been cured by Licensee within [\*\*] days (the “Notice Period”) after receipt of such notice provided that the Parties have worked together in good faith to try to cure such breach, then, The Regents will have the right to immediately terminate this Agreement by providing a written Notice of Termination to the Licensee, *provided, however*, that the stated period will be extended an additional [\*\*] days (“Notice Extension Period”) during which the Parties will work together in good faith to try to cure such breach if all of the following criteria are satisfied: (i) such breach is not susceptible of cure

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within the stated initial [\*\*] day Notice Period as evidenced by written tangible records; (ii) Licensee has submitted a cure plan that is reasonably acceptable to The Regents; and (iii) Licensee uses diligent, good faith efforts to cure such breach. If such breach is not cured during such [\*\*] day extension, The Regents will have the right to immediately terminate this Agreement by providing a written Notice of Termination to the Licensee. For the avoidance of doubt, non-monetary breach includes failure of Licensee to: (i) perform obligations described in Sections 8.1 - 8.5; and (ii) submit reports in accordance with Article 9 (Progress Reports).

11.3 This Agreement will automatically terminate without the obligation to provide written notice as set forth in Sections 11.1 or 11.2 upon (a) the bankruptcy, dissolution or winding up of the Licensee, or (b) the making or seeking to make or arrange an assignment for the benefit of creditors of the Licensee, or (c) the initiation of proceedings in voluntary or involuntary bankruptcy, or (d) the appointment of a receiver or trustee of the Licensee’s property in each case (a) to (d) that is not discharged within one hundred and twenty (120) days.

## 12. TERMINATION BY LICENSEE

12.1 **Termination At Will.** The Licensee has the right at any time to terminate this Agreement by providing a notice of termination to The Regents. Termination of this Agreement will be effective sixty (60) days from the effective date of such notice.

12.2 **Default.** Upon any material breach of this Agreement by The Regents, Licensee, in addition to any other remedy available at law or equity, shall have the right to provide notice to The Regents of such material breach. In the event such material breach has not been cured by The Regents within [\*\*] days after receipt of such notice, then, Licensee shall have the right to terminate this Agreement by providing The Regents with [\*\*] days written notice.

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## 13. CONSEQUENCES OF TERMINATION

13.1 Any termination or expiration of either this Agreement and the Collaboration Agreement will not affect the rights and obligations set forth in the following Articles:

### Data License Agreement

Article 1  
Section 2.4  
Section 6.7  
Article 7  
Article 10  
Article 13  
Article 14  
Article 15  
Section 16.2

Definitions  
Sublicense survival  
Late Payments  
Books and Records  
Term of the Agreement  
Consequences of Termination  
Use of Names and Trademarks  
Notices  
Indemnification

### Collaboration Agreement

Article 1  
Article 8  
Section 17.2  
Article 18  
Article 21  
Article 24

Definitions  
Confidentiality  
Limited Warranty  
Limitation of Liability  
Notices  
Governing Law and Jurisdiction

13.2 The termination or expiration of this Agreement will not relieve the Licensee of its obligation to pay any fees, Third Party License Fees and Earned Royalties, or any other payments owed to The Regents at the time of such termination or expiration and will not impair any accrued right of The Regents, including the right to receive Third Party License Fees and Earned Royalties in accordance with Articles 4 (Payments In Respect Of Third Party Licenses), 5 (Earned Royalties) and 13.3 (Disposition of Identified Products Upon Termination).

13.3 Upon termination (but not expiration) of this Agreement, within a period of [\*\*] days after the date of termination, the Licensee and any Third Party Licensee are entitled to (i) dispose of all previously made or partially made Identified Products, but no more and (ii) provided that

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the Sale or use of such Identified Products are subject to the terms of this Agreement, including, but not limited to, the rendering of reports and payment of Earned Royalties, Third Party License Fees and any other payments therefor required under this Agreement. The Licensee and any Third Party Licensee will not otherwise make, use, Sell, offer for Sale or import Identified Products after the date of termination.

#### 14. USE OF NAMES AND TRADEMARKS

14.1 Nothing contained in this Agreement will be construed as conferring any right to either party to use in advertising, publicity or other promotional activities any name, trade name, trademark or other designation of the other party (including a contraction, abbreviation or simulation of any of the foregoing). Without the Licensee's consent, The Regents may list Licensee's name as a licensee of technology from The Regents without further identifying the technology. Unless required by law or unless consented to in writing by Director, UCSF Office of Technology Management, the use by the Licensee of the name "The Regents of the University of California" or the name of any campus of the University of California in advertising, publicity or other promotional activities is expressly prohibited.

#### 15. NOTICES

15.1 Any notice or payment required to be given to either Party under this Agreement will be in writing and will be deemed to have been properly given and to be effective as of the date specified below if delivered to the respective address given below or to another address as designated by written notice given to the other Party:

15.1.1 on the date of delivery if delivered in person;

15.1.2 on the date of mailing if mailed by first-class certified mail, postage paid; or

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15.1.3 on the date of mailing if mailed by any global express carrier service that requires the recipient to sign the documents demonstrating the delivery of such notice or payment.

In the case of Licensee:

uniQure B.V.,  
Meibergdreef 61,  
1105BA Amsterdam,  
The Netherlands

Attention: Chief Executive Officer

In the case of The Regents:

For notices:

Office of Technology Management  
University of California, San Francisco  
185 Berry Street, Suite 4603  
San Francisco, CA 94107  
Attention: Director  
RE: UC Control No. 2012-01-0063

For remittance of payments:

Innovation Alliances & Services  
Attn: Accounts Receivable  
Reference: UC Control No. 2012-01-0063  
UC Office of the President  
1111 Franklin Street, 5th Floor  
Oakland, CA 94607-5200

#### 16. MISCELLANEOUS

16.1 This Agreement shall be subject to and governed in accordance with the following provisions outlined in the Collaboration Agreement: Sections 1 (DEFINITIONS), 8 (CONFIDENTIALITY), 9 (PUBLICATION), 17 (WARRANTIES), and 18 (LIMITATION OF LIABILITY). For avoidances of doubt, indemnification of this Agreement will be governed by Section 16.2 (Indemnification) as outlined below.

16.2.1 Licensee will, and will require its Third Party Licensees to, indemnify, hold harmless and defend The Regents, and their officers and employees ("The Regents Indemnified

Parties"), against any and all claims, suits, losses, damage, costs, fees and expenses ("Losses") resulting from any Third Party claim (including but not limited to, any product liability) arising out of the use by the Licensee or any Third Party Licensee of the Data, Know-How, Clinical Trial Data, and Project Know how or the manufacturing, use, Sale or other disposition of Identified Products by Licensee or any Third Party Licensee, except, in each case, in proportion to, and to the extent that, such Losses are the result of The Regents' gross negligence, willful misconduct or material breach of The Regents' obligations under this Agreement.

16.2.2 In the event of any such claim against The Regents Indemnified Parties by a Third Party, The Regents shall promptly notify Licensee in writing of the claim (provided, however, that any failure or delay to notify shall not excuse any obligation of Licensee except to the extent Licensee is actually prejudiced thereby) and Licensee shall solely manage and control, at its sole expense, the defense of the claim and its settlement; provided, however, that Licensee shall not settle any such claim without the prior written consent of The Regents which shall not be unreasonably withheld or delayed if such settlement does not include a complete release from liability or if such settlement would involve undertaking an obligation (including the payment of money by an The Regents Indemnified Party), would bind The Regents Indemnified Party, or includes any admission of wrongdoing or that in any way alters any intellectual property or proprietary right of The Regents. The Regents Indemnified Parties shall cooperate with Licensee and may, at their option, be represented in any such action or proceeding by counsel of their own choosing.

16.2.3 Except to the extent prohibited by law, The Regents assume all liability for damages which may arise from its own use of the Data.

16.2.4 Licensee shall at all times comply, through insurance or self-insurance, with all statutory workers' compensation and employers' liability requirements covering any and all of its employees with respect to activities performed under this Agreement. In addition to the foregoing, Licensee shall maintain, during the term of this Agreement, comprehensive general liability insurance, including products liability insurance, with reputable and financially secure insurance carrier(s) to cover the activities of Licensee.

16.2.5 The Regents shall at all times comply, through insurance or self-insurance, with all statutory workers' compensation and employers' liability requirements covering any and all employees with respect to its activities performed under this Agreement, as well as self-insurance for all of its own activities under this Agreement and its own activities related to the Clinical Trial.

16.3 Assignment. Except as expressly provided hereunder, neither this Agreement nor any rights or obligations hereunder may be assigned or otherwise transferred by either Party without the prior written consent of the other Party, which consent shall not be unreasonably withheld; provided, however, that either Party may assign this Agreement and its rights and obligations hereunder without the other Party's consent:

16.3.1 in connection with the transfer or sale of all or substantially all of the business of such Party relating to Products to a Third Party, whether by merger, sale of stock, sale of assets or otherwise, provided that in the event of a transaction (whether this Agreement is actually assigned or is assumed by the acquiring party by operation of law (*e.g.*, in the context of a reverse triangular merger)), intellectual property rights of the acquiring party in such transaction (if other than one of the Parties to this Agreement) shall not be included in the intellectual property rights licensed under this Agreement; or

16.3.2 to an Affiliate, provided that the assigning Party shall remain liable and responsible to the non-assigning Party hereto for the performance and observance of all such duties and obligations by such Affiliate; and

16.3.3 provided further that such assignee or transferee promptly agrees to be bound by the terms and conditions of this Agreement and signs The Regents' standard substitution of party letter (the form of which is attached hereto as Appendix B). This Agreement is binding upon and will inure to the benefit of The Regents, its successors and assigns.

This Agreement shall be binding upon successors and permitted assigns of the Parties. Any assignment not in accordance with this Section 16.3 will be null and void.

16.4 Further Actions. Each Party agrees to execute, acknowledge and deliver such further instruments, and to do all such other acts, as may be necessary or appropriate in order to carry out the purposes and intent of the Agreement.

16.5 Force Majeure. Neither Party shall be liable to the other for delay or failure in the performance of the obligations on its part contained in this Agreement if and to the extent that such failure or delay is due to circumstances beyond its control which it could not have avoided by the exercise of reasonable diligence. It shall notify the other Party promptly should such circumstances arise, giving an indication of the likely extent and duration thereof, and shall use all Commercially Reasonable Efforts to resume performance of its obligations as soon as practicable, *provided, however*, that neither Party shall be required to settle any labour dispute or disturbance.

16.6 Counterparts. This Agreement may be executed in two counterparts, each of which need not contain the signature of more than one Party but all such counterparts taken together shall constitute one and the same agreement.

16.7 Nothing in this Agreement grants by implication, estoppels, or otherwise any rights to the intellectual property of The Regents, except as explicitly set forth herein.

16.8 Interpretation. Except where the context requires otherwise, whenever used the singular includes the plural, the plural includes the singular, the use of any gender is applicable to all genders and the word “or” has the inclusive meaning represented by the phrase “and/or”. Whenever this Agreement refers to a number of days, unless otherwise specified, such number refers to calendar days. The headings of this Agreement are for convenience of reference only and do not define, describe, extend or limit the scope or intent of this Agreement or the scope or intent of any provision contained in this Agreement. The term “including” or “includes” as used in this Agreement means including, without limiting the generality of any description preceding such term. The wording of this Agreement shall be deemed to be the wording mutually chosen by the Parties and no rule of strict construction shall be applied against any Party.

16.9 This Agreement is not binding on the Parties until it has been signed below on behalf of each party. It is then effective as of the Effective Date.

16.10 No amendment or modification of this Agreement is valid or binding on the Parties unless made in writing and signed on behalf of each party.

16.11 This Agreement and the Collaboration Agreement embodies the entire understanding of the Parties and supersedes all previous communications, representations or understandings, either oral or written, between the Parties relating to the subject matter hereof. The Confidentiality Agreement dated June 6, 2011 is hereby superseded.

16.12 In case any of the provisions contained in this Agreement is held to be invalid, illegal or unenforceable in any respect, such invalidity, illegality or unenforceability will not affect any

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other provisions of this Agreement and this Agreement will be construed as if such invalid, illegal or unenforceable provisions had never been contained in it.

16.13 No provisions of this Agreement are intended or shall be construed to confer upon or give to any person or entity other than The Regents and the Licensee any rights, remedies or other benefits under, or by reason of, this Agreement.

16.14 In performing their respective duties under this Agreement, each of the Parties will be operating as an independent contractor. Nothing contained herein will in any way constitute any association, partnership, or joint venture between the Parties hereto, or be construed to evidence the intention of the Parties to establish any such relationship. Neither party will have the power to bind the other party or incur obligations on the other party’s behalf without the other party’s prior written consent.

16.15 Existing Patent Rights. Pursuant to section 15.1 of the Collaboration Agreement, the list of Patents known to the Industry Contracts Officer and the Technology Licensing Officer which are owned or Controlled by the University as of the Effective Date are attached hereto as Appendix C.

16.16 This Agreement includes the attached Appendices A, B and C.

IN WITNESS WHEREOF, both The Regents and the Licensee have executed this Agreement, in duplicate originals, by their respective and duly authorized officers on the day and year written.

**uniQure biopharma B.V.**

**THE REGENTS OF THE UNIVERSITY OF CALIFORNIA**

By: /s/ PJ Morgan  
(Signature)

By: /s/ Joel B. Kirschbaum  
(Signature)

Name: PJ Morgan  
(Please Print)

Name: Joel B. Kirschbaum  
(Please Print)

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Title: CFO

Title: UCSF Office of Technology Management

Date: 13 June 2012

Date: 6/13/12

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APPENDIX A  
Product Release Specifications

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APPENDIX A  
PRODUCT RELEASE SPECIFICATION

Center for Cellular and Molecular Therapeutics, The Children’s Hospital of Philadelphia  
3615 Civic Center Boulevard, Philadelphia, PA 19104

Certificate of Analysis  
AAV2-hGDNF, Lot A2FP1-1003C

Date of Manufacture: 12 OCT 2010  
Container / Closure: 1.5mL cryovial (Nalgene 500-1020)

Total number of vials: 13 7  
Nominal Fill Volume: 1mL

Attribute	Test Method		Testing Facility/ Study number	Specification	Result
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CofA AAV2-hGDNF Lot A2FP1-1003C

APPENDIX A  
PRODUCT RELEASE SPECIFICATION

Center for Cellular and Molecular Therapeutics, The Children’s Hospital of Philadelphia  
3615 Civic Center Boulevard, Philadelphia, PA 19104

Certificate of Analysis  
AAV2-hGDNF, Lot A2FP1-1003C

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APPENDIX A  
PRODUCT RELEASE SPECIFICATION

Center for Cellular and Molecular Therapeutics, The Children’s Hospital of Philadelphia  
3615 Civic Center Boulevard, Philadelphia, PA 19104

Certificate of Analysis  
AAV2-hGDNF, Lot A2FP1-1003C

[**]					
[**]	[**]	[**]	[**]	[**]	[**]
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**[\*\*]**

By: /s/ illegible  
Assistant Director, QA/QC or Designee

Date: 06 Jan 2012

/s/ illegible  
Assistant Director, QA/QC, Clinical Vector Cor Date: 06 Jan 2012

/s/ illegible  
Director, Clinica Vector Core Date: 06 Jan 2012

/s/ illegible  
Director, Clinica Vector Core Date: 06 Jan 2012

By: \_\_\_\_\_

Date: \_\_\_\_\_

Date: \_\_\_\_\_

## Patents

[illegible]



## UNIQUE

## LOAN AND SECURITY AGREEMENT

THIS LOAN AND SECURITY AGREEMENT is made and dated as of June 13, 2013 and is entered into by and among (i) UNIQUE BIOPHARMA B.V., a private limited liability company incorporated and existing under the laws of the Netherlands, having its corporate seat at Amsterdam, the Netherlands and registered at the trade register of the Chamber of Commerce for Amsterdam under number 34275365 (“**uniQure**”), (ii) UNIQUE IP B.V., a private limited liability company incorporated and existing under the laws of the Netherlands, having its corporate seat at Amsterdam, the Netherlands and registered at the trade register of the Chamber of Commerce for Amsterdam under number 34275369 (“**uniQure IP**”), (iii) each of the subsidiaries of uniQure identified on the Schedule 1 hereto and the signature pages hereof (uniQure, uniQure IP and such subsidiaries are hereinafter collectively referred to as “**Borrower**”), (iv) UNIQUE B.V., a private limited liability company incorporated and existing under the laws of the Netherlands, having its corporate seat at Amsterdam, the Netherlands and registered at the trade register of the Chamber of Commerce for Amsterdam under number 54385229, solely a party hereto for purposes of Sections 2.6 and 7.15 (“**uniQure Holdings**”) and (v) HERCULES TECHNOLOGY GROWTH CAPITAL, INC., a Maryland corporation (“**Lender**”).

## RECITALS

A. Borrower has requested Lender to make available to Borrower a term loan (the “**Term Loan Advance**”) in the principal amount of Ten Million Dollars (\$10,000,000) (the “**Term Loan Amount**”), to be used for general corporate purposes;

B. Lender is willing to make the Term Loan Advance on the terms and conditions set forth in this Agreement.

## AGREEMENT

NOW, THEREFORE, Borrower and Lender agree as follows:

SECTION 1. DEFINITIONS AND RULES OF CONSTRUCTION

1.1 Unless otherwise defined herein, the following capitalized terms shall have the following meanings:

“**Account Control Agreement(s)**” means any agreement entered into by and among the Lender, US Borrower and a third party bank or other institution (including a Securities Intermediary) in which US Borrower maintains a Deposit Account or an account holding Investment Property and which grants Lender a perfected first priority security interest in the subject account or accounts.

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“**ACH Authorization**” means the ACH Debit Authorization Agreement in substantially the form of Exhibit H.

“**Additional End of Term Charge**” has the meaning given to it in Section 2.6.

“**Advance**” means the Term Loan Advance.

“**Advance Date**” means the funding date of the Advance.

“**Advance Request**” means a request for the Advance submitted by Borrower to Lender in substantially the form of Exhibit A.

“**Agreement**” means this Loan and Security Agreement, as amended from time to time.

“**Amortization Date**” means [April 1, 2014]; provided that in the event of the occurrence of the Equity Event, such date shall be extended to [October 1, 2014](1).

“**Assignee**” has the meaning given to it in Section 11.12.

“**Borrower Products**” means all products, software, service offerings, technical data or technology currently being designed, manufactured or sold by Borrower or which Borrower intends to sell, license, or distribute in the future including any products or service offerings under development, collectively, together with all products, software, service offerings, technical data or technology that have been sold, licensed or distributed by Borrower since its incorporation.

“**Business Day**” is any day that is not a Saturday, Sunday or a day on which Lender is closed.

“**Cash**” means all cash and liquid funds.

“**Change in Control**” means any (i) reorganization, recapitalization, consolidation or merger (or similar transaction or series of related transactions) of uniQure Holdings, Borrower or any Subsidiary, sale or exchange of outstanding shares (or similar transaction or series of related transactions) of uniQure Holdings, Borrower or any Subsidiary in which the holders of uniQure Holdings’ Borrower’s or Subsidiary’s outstanding shares immediately before consummation of such transaction or series of related transactions do not, immediately after consummation of such transaction or series of related transactions, retain shares representing more than fifty percent (50%) of the voting power of the surviving entity of such transaction or series of related transactions (or the parent of such surviving entity if such surviving entity is wholly owned by such parent), in each case without regard to whether uniQure Holdings, Borrower or Subsidiary is the surviving entity, or (ii) sale or issuance by uniQure Holdings, Borrower of new shares of Preferred Stock of uniQure Holdings or Borrower to investors, none of whom are current investors in uniQure Holdings or Borrower, and such new shares of Preferred Stock are senior to all existing Preferred Stock and common stock of uniQure Holdings or Borrower with respect to

(1) Dates to be confirmed prior to funding

liquidation preferences, and the aggregate liquidation preference of the new shares of Preferred Stock is more than fifty percent (50%) of the aggregate liquidation preference of all shares of Preferred Stock of uniQure Holdings or Borrower; provided, however, an Initial Public Offering shall not constitute a Change in Control.

**“Closing Date”** means the date of this Agreement.

**“Collateral”** means the property described in Section 3.

**“Collateral Documents”** means the security documents described in Section 3.

**“Commitment Fee”** means \$45,000, which fee has been received by Lender and shall be deemed fully earned on the Closing Date, regardless of the early termination of this Agreement.

**“Confidential Information”** has the meaning given to it in Section 11.11.

**“Contingent Obligation”** means, as applied to any Person, any direct or indirect liability, contingent or otherwise, of that Person with respect to (i) any indebtedness, lease, dividend, letter of credit or other obligation of another, including any such obligation directly or indirectly guaranteed, endorsed, co-made or discounted or sold with recourse by that Person, or in respect of which that Person is otherwise directly or indirectly liable; (ii) any obligations with respect to undrawn letters of credit, corporate credit cards or merchant services issued for the account of that Person; and (iii) all obligations arising under any interest rate, currency or commodity swap agreement, interest rate cap agreement, interest rate collar agreement, or other agreement or arrangement designated to protect a Person against fluctuation in interest rates, currency exchange rates or commodity prices; provided, however, that the term “Contingent Obligation” shall not include endorsements for collection or deposit in the ordinary course of business. The amount of any Contingent Obligation shall be deemed to be an amount equal to the stated or determined amount of the primary obligation in respect of which such Contingent Obligation is made or, if not stated or determinable, the maximum reasonably anticipated liability in respect thereof as determined by such Person in good faith; provided, however, that such amount shall not in any event exceed the maximum amount of the obligations under the guarantee or other support arrangement.

**“Copyright License”** means any written agreement granting any right to use any Copyright or Copyright registration, now owned or hereafter acquired by Borrower or in which Borrower now holds or hereafter acquires any interest.

**“Copyrights”** means all copyrights, whether registered or unregistered, held by the Borrower pursuant to the laws of the Netherlands, or of any other country.

**“Deposit Accounts”** means any “deposit accounts,” including any checking account, savings account, or certificate of deposit and any deposit account as defined in the UCC.

**“End of Term Charge”** is defined in Section 2.5

**“Equity Event”** means confirmation by Lender that Borrower has received, after the Closing Date but on or prior to [April 1, 2014](2), unrestricted and unencumbered net cash proceeds in an amount of at least forty million Dollars (\$40,000,000.00) from (a) the issuance of new equity or convertible debt securities with investors reasonably acceptable to Lender, or (b) net upfront payments (such payments consisting of any combination of cash or cash received for the purchase of the Borrower’s equity or convertible debt with investors reasonably acceptable to Lender).

**“Event of Default”** has the meaning given to it in Section 9.

**“Facility Charge”** means one and one-half of one percent (1.25%) of the Term Loan Amount.

**“Financial Statements”** has the meaning given to it in Section 7.1.

**“Funding Documents”** means the following: (i) a certificate of good standing for US Borrower from its state of incorporation and from all other US jurisdictions in which it does business, and, if applicable under the laws of any non-US jurisdiction, a certificate of good standing or the equivalent for Borrower and US Borrower from all non-US jurisdictions in which such entity does business, in each case where the failure to be qualified to do business would have a Material Adverse Effect; (ii) completed Schedules and Exhibits to this Agreement; (iii) executed originals of the following: (x) the Account Control Agreements, and any other documents executed in connection with the Secured Obligations or the transactions contemplated hereby, as the same may from time to time be amended, modified, supplemented or restated and (y) the Perfection Certificate; (iv) legal opinion of Lender’s counsel; (v) the insurance policies and/or endorsements required pursuant to Section 6.1 hereof; (vi) documents, releases, terminations, and other instruments as may be necessary or proper to release any creditor’s Lien in the Intellectual Property of Borrower including, without limitation, UCC financing statement amendments and appropriate filings with any appropriate register or authority in any jurisdiction; and (vii) and all other documents and instruments reasonably required by Lender to effectuate the transactions contemplated hereby or to create and perfect the Liens of Lender with respect to all Collateral, in all cases in form and substance reasonably acceptable to Lender.

**“IFRS”** are the International Financial Reporting Standards, a collection of guidelines and rules set by the International Accounting Standards Board ([www.iasb.org](http://www.iasb.org)) which are applicable to the circumstances as of the date of determination.

**“Indebtedness”** means indebtedness of any kind, including (a) all indebtedness for borrowed money or the deferred purchase price of property or services (excluding trade credit entered into in the ordinary course of business due within sixty (60) days), including reimbursement and other obligations with respect to surety bonds and letters of credit, (b) all obligations evidenced by notes, bonds, debentures or similar instruments, (c) all capital lease obligations, and (d) all Contingent Obligations.

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(2) Date to be confirmed prior to funding

**“Initial Public Offering”** means the initial firm commitment underwritten offering of uniQure Holdings common stock pursuant to a registration statement under the Securities Act of 1933 filed with and declared effective by the Securities and Exchange Commission.

**“Insolvency Proceeding”** is any proceeding by or against any Person under the Dutch Bankruptcy Act, or any other bankruptcy or insolvency law, including assignments for the benefit of creditors, compositions, extensions generally with its creditors, or proceedings seeking reorganization, arrangement, or other relief.

**“Intellectual Property”** means any and all intellectual property rights in any country or jurisdiction, including but not limited to all of Borrower’s Copyrights; Trademarks; Patents; Licenses; trade secrets and inventions; mask works, utility models, layout-designs (topographies) of integrated circuits, know-how, industrial designs, neighbouring rights, database rights or other rights in compilations of data, trade names, internet domain names, plant variety rights and any and all rights of a similar nature, either (i) now known, contemplated or unforeseen, (ii) having a statutory basis or existing under equity, common law or otherwise, (iii) registered, deposited, filed or not, and including any and all rights in connection with applications for or rights to apply for or acquire any and all of such rights.

**“Investment”** means any beneficial ownership (including stock, partnership or limited liability company interests) of or in any Person, or any loan, advance or capital contribution to any Person or the acquisition of all, or substantially all, of the assets of another Person.

**“Joinder Agreements”** means for each Subsidiary, a completed and executed Joinder Agreement in substantially the form attached hereto as Exhibit G.

**“Lender”** has the meaning given to it in the preamble to this Agreement.

**“License”** means any Copyright License, Patent License, Trademark License or other license of rights or interests.

**“Lien”** means any mortgage, deed of trust, pledge, hypothecation, assignment for security, security interest, encumbrance, levy, lien or charge of any kind, whether voluntarily incurred or arising by operation of law or otherwise, against any property, any conditional sale or other title retention agreement, and any lease in the nature of a security interest.

**“Loan”** means the Advance made under this Agreement.

**“Loan Documents”** means this Agreement, the Notes (if any), the ACH Authorization, the Account Control Agreements, the Joinder Agreements, all UCC Financing Statements, the Warrant Agreement (if applicable), any intellectual property security agreement, and any other documents executed in connection with the Secured Obligations or the transactions contemplated hereby, as the same may from time to time be amended, modified, supplemented or restated.

**“Material Adverse Effect”** means a material adverse effect upon: (i) the business, operations, properties, assets or condition (financial or otherwise) of Borrower and its Subsidiaries, taken as a whole, other than in and of itself (x) the expenditure of cash in the ordinary course, or (y) adverse results of a clinical trial or program or the denial, delay or

limitation of approval of, or taking of any other regulatory action by, the United States Food and Drug Administration or any other governmental entity with respect to any biologic product or drug; or (ii) the ability of Borrower to perform the Secured Obligations when due in accordance with the terms of the Loan Documents, or the ability of Lender to enforce any of its rights or remedies with respect to the Secured Obligations; or (iii) the Collateral or Lender’s Liens on the Collateral or the priority of such Liens

**“Maximum Rate”** shall have the meaning assigned to such term in Section 2.2.

**“Note(s)”** means a promissory note or promissory notes to evidence Lender’s Loans.

**“Patent License”** means any written agreement granting any right with respect to any invention on which a Patent is in existence or a Patent application is pending, in which agreement Borrower now holds or hereafter acquires any interest.

**“Patents”** means any patent in the Netherlands or in any other country, all registrations and recordings thereof, and all applications for patents of, or rights corresponding thereto, in the Netherlands or any other country.

**“Permitted Indebtedness”** means: (i) Indebtedness of Borrower in favor of Lender arising under this Agreement or any other Loan Document; (ii) Indebtedness existing on the Closing Date which is disclosed in Schedule 1A; (iii) Indebtedness of up to \$250,000 outstanding at any time secured by a Lien described in clause (vii) of the defined term “Permitted Liens,” provided such Indebtedness does not exceed the lesser of the cost or fair market value of the equipment financed with such Indebtedness; (iv) Indebtedness to trade creditors incurred in the ordinary course of business, including Indebtedness incurred in the ordinary course of business with corporate credit cards; (v) Indebtedness that also constitutes a Permitted Investment; (vi) Subordinated Indebtedness; (vii) reimbursement obligations in connection with letters of credit that are secured by cash or cash equivalents and issued on behalf of the Borrower or a Subsidiary thereof in an amount not to exceed \$200,000 at any time outstanding, (viii) other Indebtedness in an amount not to exceed \$100,000 at any time outstanding, and (ix) extensions, refinancings and renewals of any items of Permitted Indebtedness, provided that the principal amount is not increased or the terms modified to impose materially more burdensome terms upon Borrower or its Subsidiary, as the case may be.

**“Permitted Investment”** means: (i) Investments existing on the Closing Date which are disclosed in Schedule 1B; (ii) (a) marketable direct obligations issued or unconditionally guaranteed by any agency or any country thereof maturing within one year from the date of acquisition thereof, (b) commercial paper maturing no more than one year from the date of creation thereof and currently having a rating of at least A-2 or P-2 from either Standard & Poor’s Corporation or Moody’s Investors Service, (c) certificates of deposit issued by any bank with assets of at least \$500,000,000 maturing no more than one year from the date of investment therein, and (d) money market accounts; (iii) repurchases of stock from former employees, directors, or consultants of Borrower under the terms of applicable repurchase agreements at the original issuance price of such securities in an aggregate amount not to exceed \$250,000 in any fiscal year, provided that no Event of Default has occurred, is continuing or would exist after giving effect to the repurchases; (iv) Investments accepted in connection with Permitted

Transfers; (v) Investments (including debt obligations) received in connection with the bankruptcy or reorganization of customers or suppliers and in settlement of delinquent obligations of, and other disputes with, customers or suppliers arising in the ordinary course of Borrower's business; (vi) Investments consisting of notes receivable of, or prepaid royalties and other credit extensions, to customers and suppliers who are not affiliates, in the ordinary course of business, provided that this subparagraph (vi) shall not apply to Investments of Borrower in any Subsidiary; (vii) Investments consisting of loans not involving the net transfer on a substantially contemporaneous basis of cash proceeds to employees, officers or directors relating to the purchase of capital stock of Borrower pursuant to employee stock purchase plans or other similar agreements approved by Borrower's board of directors; (viii) Investments consisting of employee travel advances, employee relocation loans and other employee loans and advances in the ordinary course of business; (ix) Investments in newly-formed Subsidiaries organized in the Netherlands or any other country, provided that such Subsidiaries enter into a Joinder Agreement promptly after their formation by Borrower and execute such other documents as shall be reasonably requested by Lender; (xi) joint ventures or strategic alliances in the ordinary course of Borrower's business consisting of the nonexclusive licensing of technology, the development of technology or the providing of technical support, provided that any cash Investments by Borrower do not exceed \$100,000 in the aggregate in any fiscal year; and (xii) other Investments that do not exceed \$250,000 in the aggregate.

**"Permitted Liens"** means any and all of the following: (i) Liens in favor of Lender; (ii) Liens existing on the Closing Date which are disclosed in Schedule 1C; (iii) Liens for taxes, fees, assessments or other governmental charges or levies, either not delinquent or being contested in good faith by appropriate proceedings; provided, that Borrower maintains adequate reserves therefor in accordance with IFRS; (iv) Liens securing claims or demands of materialmen, artisans, mechanics, carriers, warehousemen, landlords and other like Persons arising in the ordinary course of Borrower's business and imposed without action of such parties; provided, that the payment thereof is not yet required; (v) Liens arising from judgments, decrees or attachments in circumstances which do not constitute an Event of Default hereunder; (vi) the following deposits, to the extent made in the ordinary course of business: deposits under worker's compensation, unemployment insurance, social security and other similar laws, or to secure the performance of bids, tenders or contracts (other than for the repayment of borrowed money) or to secure indemnity, performance or other similar bonds for the performance of bids, tenders or contracts (other than for the repayment of borrowed money) or to secure statutory obligations (other than liens arising under environmental liens) or surety or appeal bonds, or to secure indemnity, performance or other similar bonds; (vii) Liens on equipment or software or other intellectual property constituting purchase money liens and liens in connection with capital leases securing Indebtedness permitted in clause (iii) of "Permitted Indebtedness"; (viii) Liens incurred in connection with Subordinated Indebtedness; (ix) leasehold interests in leases or subleases and licenses granted in the ordinary course of business and not interfering in any material respect with the business of the licensor; (x) Liens in favor of customs and revenue authorities arising as a matter of law to secure payment of custom duties that are promptly paid on or before the date they become due; (xi) Liens on insurance proceeds securing the payment of financed insurance premiums that are promptly paid on or before the date they become due (provided that such Liens extend only to such insurance proceeds and not to any other property or assets); (xii) statutory and common law rights of set-off and other similar rights as to deposits

of cash and securities in favor of banks, other depository institutions and brokerage firms; (xiii) easements, zoning restrictions, rights-of-way and similar encumbrances on real property imposed by law or arising in the ordinary course of business so long as they do not materially impair the value or marketability of the related property; (xiv) Liens on cash or cash equivalents securing obligations permitted under clause (vii) of the definition of Permitted Indebtedness; and (xv) Liens incurred in connection with the extension, renewal or refinancing of the indebtedness secured by Liens of the type described in clauses (i) through (xi) above; provided, that any extension, renewal or replacement Lien shall be limited to the property encumbered by the existing Lien and the principal amount of the indebtedness being extended, renewed or refinanced (as may have been reduced by any payment thereon) does not increase.

**"Permitted Transfers"** means (i) sales of inventory in the normal course of business, (ii) exclusive licenses and similar arrangements for the use of Intellectual Property in the ordinary course of business that could not result in a legal transfer of title of the licensed property (iii) dispositions of worn-out, obsolete or surplus equipment that is, in the reasonable judgment of Borrower, no longer economically practicable to maintain or useful in the ordinary course of business of Borrower, (iv) other Transfers of assets having a fair market value of not more than \$250,000 in the aggregate in any fiscal year and (v) the entering into the commercialization agreement, the co-development and license agreement and any other related documents by and among uniQure and Chiesi Farmaceutici S.p.A (the "**Chiesi transaction**").

**"Person"** means any individual, sole proprietorship, partnership, joint venture, trust, unincorporated organization, association, corporation, limited liability company, institution, other entity or government.

**"Preferred Stock"** means at any given time any equity security issued by Borrower that has any rights, preferences or privileges senior to uniQure Holdings' or Borrower's common stock.

**"Prepayment Charge"** shall have the meaning assigned to such term in Section 2.4.

**"Prime Rate"** means the "prime rate" as reported in *The Wall Street Journal*, and if not reported, then the prime rate most recently reported in *The Wall Street Journal*.

**"Secured Obligations"** means Borrower's obligations under this Agreement and any Loan Document, including any obligation to pay any amount now owing or later arising.

**"Subordinated Indebtedness"** means Indebtedness subordinated to the Secured Obligations in amounts and on terms and conditions satisfactory to Lender in its sole discretion.

**"Subsequent Financing"** means the closing of any Borrower financing which becomes effective after the Closing Date and results in aggregate proceeds to Borrower of at least [Ten Million Dollars (\$10,000,000)].

**"Subsidiary"** means an entity, whether corporate, partnership, limited liability company, joint venture or otherwise, in which Borrower owns or controls 50% or more of the outstanding voting securities, including each entity listed on Schedule 1 hereto.

“**Term Loan Advance**” is defined in Recital A hereof.

“**Term Loan Amount**” is defined in Recital A hereof.

“**Term Loan Interest Rate**” means for any day, a floating per annum rate of interest equal to the greater of either (i) eleven and eighty-five one-hundredths of one percent (11.85%), or (ii) the sum of (A) eleven and eighty-five one-hundredths of one percent (11.85%), plus (B) the Prime Rate minus three and one quarter of one percent (3.25%). The Term Loan Interest Rate will change from time to time on the day the Prime Rate changes.

“**Term Loan Maturity Date**” means [October 1, 2016](3).

“**Trademark License**” means any written agreement granting any right to use any Trademark or Trademark registration, now owned or hereafter acquired by Borrower or in which Borrower now holds or hereafter acquires any interest.

“**Trademarks**” means all trademarks (registered, common law or otherwise) and any applications in connection therewith, including registrations, recordings and applications with any appropriate register or authority in any jurisdiction.

“**UCC**” means the Uniform Commercial Code as the same is, from time to time, in effect in the State of California; provided, that in the event that, by reason of mandatory provisions of law, any or all of the attachment, perfection or priority of, or remedies with respect to, Lender’s Lien on any Collateral is governed by the Uniform Commercial Code as the same is, from time to time, in effect in a jurisdiction other than the State of California, then the term “UCC” shall mean the Uniform Commercial Code as in effect, from time to time, in such other jurisdiction solely for purposes of the provisions thereof relating to such attachment, perfection, priority or remedies and for purposes of definitions related to such provisions.

“**US Borrower**” means [ ] identified on the Schedule 1 hereto (4);

“**Warrant Agreement**” is defined in Section 2.6

Unless otherwise specified, all references in this Agreement or any Annex or Schedule hereto to a “Section,” “subsection,” “Exhibit,” “Annex,” or “Schedule” shall refer to the corresponding Section, subsection, Exhibit, Annex, or Schedule in or to this Agreement. Unless otherwise specifically provided herein, any accounting term used in this Agreement or the other Loan Documents shall have the meaning customarily given such term in accordance with IFRS, and all financial computations hereunder shall be computed in accordance with IFRS, consistently applied. Unless otherwise defined herein or in the other Loan Documents, terms that are used herein or in the other Loan Documents and defined in the UCC shall have the meanings given to them in the UCC.

(3) Date to be confirmed prior to closing.

(4) To be confirmed that the new US subsidiary will be incorporated prior to closing.

## **SECTION 2. THE LOAN**

### **2.1 Term Loan.**

(a) **Advance.** Subject to the terms and conditions of this Agreement, Borrower may request a Term Loan Advance in the principal amount of the Term Loan Amount to be funded on the Closing Date. Proceeds of the Advance shall be deposited into an account that is subject to a security interest in favor of Lender, perfected by an Account Control Agreement.

(b) **Advance Request.** To obtain the Term Loan Advance, Borrower shall complete, sign and deliver to Lender an Advance Request (at least five Business Days before the Advance Date). Lender shall fund the Term Loan Advance in the manner requested by the Advance Request provided that each of the conditions precedent to such Term Loan Advance is satisfied as of the requested Advance Date.

(c) **Interest.** The principal balance of each Term Loan Advance shall bear interest thereon from such Advance Date at the Term Loan Interest Rate based on a year consisting of 360 days, with interest computed daily based on the actual number of days elapsed. The Term Loan Interest Rate will float and change on the day the Prime Rate changes from time to time.

(d) **Payment.** Borrower will pay interest on the Term Loan Advance on the first Business Day of each month, beginning the month after the Advance Date. Commencing on the Amortization Date, and continuing on the first Business Day of each month thereafter, Borrower shall repay the aggregate principal balance of Term Loan Advance that is outstanding in 30 equal monthly installments of principal and interest (mortgage style). The entire outstanding principal balance of the Term Loan Advance and all accrued but unpaid interest hereunder, and all other Secured Obligations with respect to the Term Loan Advance, shall be due and payable on Term Loan Maturity Date. Borrower shall make all payments under this Agreement without setoff, recoupment or deduction and regardless of any counterclaim or defense. Lender will initiate debit entries to the Borrower’s account as authorized on the ACH Authorization on each payment date of all periodic obligations payable to Lender under the Term Loan Advance. Once repaid, the Term Loan Advance or any portion thereof may not be reborrowed.

2.2 **Maximum Interest.** Notwithstanding any provision in this Agreement or any other Loan Document, it is the parties’ intent not to contract for, charge or receive interest at a rate that is greater than the maximum rate permissible by law that a court of competent jurisdiction shall deem applicable hereto (which under the laws of the State of California shall be deemed to be the laws relating to permissible rates of interest on commercial loans) (the “Maximum Rate”). If a court of competent jurisdiction shall finally determine that Borrower has actually paid to Lender an amount of interest in excess of the amount that would have been payable if all of the Secured Obligations had at all times borne interest at the Maximum Rate, then such excess interest actually paid by Borrower shall be applied as follows: first, to the payment of the Secured Obligations consisting of the outstanding principal of the Term Loan Advance; second, after all principal is repaid, to the payment of Lender’s accrued interest, costs, expenses, professional fees and any other Secured Obligations; and third, after all Secured Obligations are repaid, the excess (if any) shall be refunded to Borrower.

2.3 **Default Interest.** In the event any payment is not paid on the scheduled payment date, an amount equal to five percent (5%) of the past due amount shall be payable on demand. [In addition, upon the occurrence and during the continuation of an Event of Default hereunder, all Secured Obligations, including principal, interest, compounded interest, and professional fees, shall bear interest at a rate per annum equal to the rate set forth in Section 2.1(c), plus five percent (5%) per annum. In the event any interest is not paid when due hereunder, delinquent interest shall be added to principal and shall bear interest on interest, compounded at the rate set forth in Section 2.1(c).

2.4 **Prepayment.** At its option upon at least five (5) Business Days prior notice to Lender, Borrower may prepay all, but not less than all, of the outstanding Advance by paying the entire outstanding principal balance, all accrued and unpaid interest thereon, all unpaid Lender's fees and expenses accrued to the date of the repayment (including, without limitation, the End of Term Charge) together with a prepayment charge equal to the following percentage of the Advance amount being prepaid: if such Advance amounts are prepaid in any of the first twelve (12) months following the Closing Date, three percent (3.00%); after twelve (12) months but prior to twenty four (24) months, two percent (2.0%); and after twenty four (24) months but prior to the Term Loan Maturity Date, one percent (1%) (each, a "**Prepayment Charge**"). Borrower agrees that the Prepayment Charge is a reasonable calculation of Lender's lost profits in view of the difficulties and impracticality of determining actual damages resulting from an early repayment of the Advances. Borrower shall prepay the outstanding amount of all principal and accrued interest through the prepayment date and all unpaid Lender's fees and expenses accrued to the date of the repayment (including the End of Term Charge) together with a Prepayment Charge upon the occurrence of a Change in Control.

2.5 **End of Term Charge.** On the earliest to occur of (i) the Term Loan Maturity Date, (ii) the date that Borrower prepays the outstanding Secured Obligations, or (iii) the date that the Secured Obligations become due and payable, Borrower shall pay Lender a charge equal \$345,000, or the increased amount in accordance with Section 2.6 (the "**End of Term Charge**"). Notwithstanding the required payment date of such charge, it shall be deemed earned by Lender as of the Closing Date.

2.6 **Additional compensation.** Parties agree that, at the option and written election of the Borrower in its sole and absolute discretion, and as additional consideration for the Term Loan Advance, either (i) the End of Term Charge payable in accordance with Section 2.5 shall be increased with an additional \$1,000,000 (the "**Additional End of Term Charge**"), as a result of which increase the total End of Term Charge payable by Borrower in accordance with Section 2.5 above shall amount to \$1,345,000, or (ii) in lieu of, and not in addition to, payment of the Additional End of Term Charge, uniQure Holdings shall execute and deliver to Lender the Warrant Agreement, representing the right to subscribe for shares in the share capital of uniQure Holdings, in substantially the form attached hereto as Schedule 2.6 (the "**Warrant Agreement**"). Borrower shall ultimately on September 29, 2013 confirm which option it prefers by giving notice in writing to Lender.

The right of Borrower to elect the form of additional compensation as set forth above will expire on the earlier of (i) September 30, 2013 or (ii) the occurrence of a Change of Control event provided that the Warrant Agreement has not yet been signed by all parties thereto. Following

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such expiration Borrower shall have deemed to have elected to increase the End of Term Charge in accordance with option (i) above.

The grant of the warrants, if so elected by Borrower, shall be effectuated by means of the execution of the Warrant Agreement ultimately within 3 Business Days following the confirmation by Borrower but in any event no later than September 30, 2013.

2.7 **Notes.** If so requested by Lender by written notice to Borrower, then Borrower shall execute and deliver to Lender (and/or, if applicable and if so specified in such notice, to any person who is an assignee of Lender pursuant to Section 11.12) (promptly after the Borrower's receipt of such notice) a Note or Notes to evidence Lender's Loans.

### **SECTION 3. SECURITY INTEREST**

3.1 As security for the prompt, complete and indefeasible payment when due (whether on the payment dates or otherwise) of all the Secured Obligations:

(a) uniQure Holdings grants to Lender a first ranking right of pledge on its shares in uniQure and uniQure IP;

(b) uniQure grants to Lender a first ranking right of pledge on its shares in its Dutch subsidiaries identified on the Schedule 1 hereto and a security interest in 100% of the capital stock of US Borrower;

(c) Borrower (excluding US Borrower) grants to Lender a first ranking right of pledge on its (a) trade, intercompany and insurance receivables; (b) movable assets and (c) Deposit Accounts; and

(d) US Borrower grants to Lender a security interest in all of US Borrower's right, title, and interest in and to the following personal property whether now owned or hereafter acquired: (a) receivables; (b) equipment; (c) fixtures; (d) general intangibles (except as described below); (e) inventory; (f) Investment property; (g) Deposit Accounts; (h) Cash; (i) Goods; and all other tangible and intangible personal property of US Borrower whether now or hereafter owned or existing, leased, consigned by or to, or acquired by, US Borrower and wherever located, and any of Borrower's property in the possession or under the control of Lender; and, to the extent not otherwise included, all Proceeds of each of the foregoing and all accessions to, substitutions and replacements for, and rents, profits and products of each of the foregoing (collectively, the "**Collateral**").

3.2 Notwithstanding anything in this Agreement or any other Loan Document to the contrary, in no event shall the Collateral include, and the Borrower shall not be deemed to have granted a security interest in: (i) Intellectual Property; provided, however, that the Collateral shall include all accounts and general intangibles that consist of rights to payment and proceeds from the sale, licensing or disposition of all or any part, or rights in, the Intellectual Property (the "Rights to Payment"); or (ii) any of the Borrower's rights or interests in or under, any license, contract, permit, instrument, security or franchise to which the Borrower is a party or any of its rights or interests thereunder to the extent, but only to the extent, that such a grant would, under

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the terms of such license, contract, permit, instrument, security or franchise, result in a breach of the terms of, or constitute a default under, such license, contract, permit, instrument, security or franchise (other than to the extent that any such term would be rendered ineffective pursuant to the UCC or any other applicable law (including the Dutch and the United States Bankruptcy Code) or principles of equity); provided, that immediately upon the ineffectiveness, lapse or termination of

any such provision the Collateral shall include, and the Borrower shall be deemed to have granted a security interest in, all the rights and interests described in the foregoing clause (ii) as if such provision had never been in effect. Notwithstanding the foregoing, if a judicial authority (including a U.S. Bankruptcy Court) holds that a security interest in the underlying Intellectual Property is necessary to have a security interest in the Rights to Payment, then the Collateral shall automatically, and effective as of the date of this Agreement, include the Intellectual Property to the extent necessary to permit perfection of Lender's security interest in the Rights to Payment.

#### **SECTION 4. CONDITIONS PRECEDENT TO LOAN**

The obligation of Lender to make the Term Loan Advance hereunder is subject to the satisfaction by Borrower of the following conditions:

4.1 Closing Documents. On or prior to the Closing Date, Borrower shall have delivered to Lender the following:

- (a) executed originals of this Agreement, the Collateral Documents and the ACH Authorization;
- (b) copies of resolutions of Borrower's board of directors and general meeting of shareholders evidencing approval of (i) the Loan and other transactions evidenced by the Loan Documents;
- (c) copies of the current articles of association of Borrower;
- (d) payment of the Facility Charge and reimbursement of Lender's current expenses reimbursable pursuant to this Agreement; and
- (e) receipt of the Funding Documents and satisfaction of all conditions precedent thereto;
- (f) Lender shall have received (i) an Advance Request for the relevant Advance as required by 2.2(b), duly executed by Borrower's Chief Executive Officer or Chief Financial Officer, and (ii) any other documents Lender may reasonably request.

(g) The representations and warranties set forth in this Agreement and in Section 5 shall be true and correct in all material respects on and as of the Advance Date with the same effect as though made on and as of such date, except to the extent such representations and warranties expressly relate to an earlier date.

(h) Borrower shall be in compliance with all the terms and provisions set forth herein and in each other Loan Document on its part to be observed or performed, and at the time

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of and immediately after such Advance no Event of Default shall have occurred and be continuing.

(i) The Advance Request shall be deemed to constitute a representation and warranty by Borrower on the Advance Date as to the matters specified in Section 4.2 and as to the matters set forth in the Advance Request.

4.2 No Default. As of the Closing Date and the Advance Date, (i) no fact or condition exists that would (or would, with the passage of time, the giving of notice, or both) constitute an Event of Default and (ii) no event that has had or could reasonably be expected to have a Material Adverse Effect has occurred and is continuing.

#### **SECTION 5. REPRESENTATIONS AND WARRANTIES OF BORROWER**

Borrower represents and warrants that:

5.1 Corporate Status. Borrower is a private limited liability company duly incorporated and existing under the laws of the Netherlands, and is duly qualified as a foreign corporation in all jurisdictions in which the nature of its business or location of its properties require such qualifications and where the failure to be qualified could reasonably be expected to have a Material Adverse Effect. Borrower's present name, former names (if any), locations, place of formation, tax identification number, organizational identification number and other information are correctly set forth in Exhibit C, as may be updated by Borrower in a written notice (including any Compliance Certificate) provided to Lender after the Closing Date. US Borrower is corporation duly organized, legally existing and in good standing under the laws of the \_\_\_\_\_, and is duly qualified as a foreign corporation in all jurisdictions in which the nature of its business or location of its properties require such qualifications and where the failure to be qualified could reasonably be expected to have a Material Adverse Effect

5.2 Collateral. Borrower owns the Collateral and the Intellectual Property, free of all Liens, except for Permitted Liens. Borrower has the power and authority to grant to Lender a Lien in the Collateral as security for the Secured Obligations.

5.3 Consents. Borrower's execution, delivery and performance of the Notes (if any), this Agreement and all other Loan Documents, (i) have been duly authorized by all necessary corporate action of Borrower, (ii) will not result in the creation or imposition of any Lien upon the Collateral, other than Permitted Liens and the Liens created by this Agreement and the other Loan Documents, (iii) do not violate any provisions of Borrower's articles of association, or any, law, regulation, order, injunction, judgment, decree or writ to which Borrower is subject and (iv) except as described on Schedule 5.3, do not violate any contract or agreement or require the consent or approval of any other Person which has not already been obtained. The individual or individuals executing the Loan Documents are duly authorized to do so.

5.4 Material Adverse Effect. No event that has had or could reasonably be expected to have a Material Adverse Effect has occurred and is continuing. Borrower is not aware of any event likely to occur that is reasonably expected to result in a Material Adverse Effect.

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5.5 Actions Before Governmental Authorities. Except as described on Schedule 5.5, there are no actions, suits or proceedings at law or in equity or by or before any governmental authority now pending or, to the knowledge of Borrower, threatened against or affecting Borrower or its property (i) which seek to prevent, enjoin, hinder or delay the transactions contemplated by the Loan Documents or (ii) as to which there is a reasonable possibility of an adverse

determination and which, if adversely determined, would reasonably be expected to, individually or in the aggregate, have a material adverse effect on Borrower's business.

5.6 Laws. Borrower, to its knowledge, is not in violation of any law, rule or regulation, or in default with respect to any judgment, writ, injunction or decree of any governmental authority, where such violation or default is reasonably expected to result in a Material Adverse Effect. Borrower, to its knowledge, is not in default in any manner under any provision of any agreement or instrument evidencing indebtedness, or any other material agreement to which it is a party or by which it is bound and for which such default would reasonably be expected to have a material adverse effect on Borrower's business.

5.7 Information Correct and Current. No information, report, Advance Request, financial statement, exhibit or schedule furnished, by or on behalf of Borrower to Lender in connection with any Loan Document or included therein or delivered pursuant thereto contained, contains or will contain any material misstatement of fact or omitted, omits or will omit to state any material fact necessary to make the statements therein, in the light of the circumstances under which they were, are or will be made, not misleading at the time such statement was made or deemed made. Additionally, any and all financial or business projections provided by Borrower to Lender shall be (i) provided in good faith and based on the most current data and information available to Borrower, (ii) the most current of such projections provided to Borrower's board of directors, and (iii) are based on reasonable assumptions not viewed as facts and that actual results during the period or periods covered by such projections and forecast may differ from the projected or forecasted results.

5.8 Tax Matters. Except as described on Schedule 5.8, (a) Borrower has filed all federal, state and local tax returns that it is required to file, (b) Borrower has duly paid or fully reserved for all taxes or installments thereof (including any interest or penalties) as and when due, which have or may become due pursuant to such returns, and (c) Borrower has paid or fully reserved for any tax assessment received by Borrower for the three (3) years preceding the Closing Date, if any (including any taxes being contested in good faith and by appropriate proceedings).

5.9 Intellectual Property Claims. Borrower is the sole owner of, or otherwise has the right to use, the Intellectual Property. Except as described on Schedule 5.9, (i) each of the material Copyrights, Trademarks and Patents is valid and enforceable, (ii) no material part of the Intellectual Property has been judged invalid or unenforceable, in whole or in part, and (iii) no claim has been made in writing to Borrower that any material part of the Intellectual Property violates the rights of any third party. Exhibit D is a true, correct and complete list of each of Borrower's Patents, registered Trademarks, registered Copyrights, and material agreements under which Borrower licenses Intellectual Property from third parties (other than shrink-wrap software licenses and other licenses for over-the-counter software), together with application or registration numbers, as applicable, owned by Borrower or any Subsidiary, in each case as of the

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Closing Date. Borrower is not in material breach of, nor has Borrower failed to perform any material obligations under, any of the foregoing contracts, licenses or agreements and, to Borrower's knowledge, no third party to any such contract, license or agreement is in material breach thereof or has failed to perform any material obligations thereunder.

5.10 Intellectual Property. Except as described on Schedule 5.10, Borrower has, or in the case of any proposed business, will have, all material rights with respect to Intellectual Property necessary in the operation or conduct of Borrower's business as currently conducted and proposed to be conducted by Borrower. Without limiting the generality of the foregoing, and in the case of Licenses, except for restrictions that are unenforceable under Division 9 of the UCC, Borrower has the right, to the extent required to operate Borrower's business, to freely transfer, license or assign Intellectual Property without condition, restriction or payment of any kind (other than license payments in the ordinary course of business) to any third party, and Borrower owns or has the right to use, pursuant to valid licenses, all software development tools, library functions, compilers and all other third-party software and other items that are necessary in the design, development, promotion, sale, license, manufacture, import, export, use or distribution of Borrower Products.

5.11 Borrower Products. Except as described on Schedule 5.11, no Intellectual Property owned by Borrower or Borrower Product has been or is subject to any actual or, to the knowledge of Borrower, threatened litigation, proceeding or outstanding decree, order, judgment, settlement agreement or stipulation that restricts in any material manner Borrower's use, transfer or licensing thereof or that may materially affect the validity, use or enforceability thereof. There is no decree, order, judgment, agreement, stipulation, arbitral award or other provision entered into in connection with any litigation or proceeding that obligates Borrower to grant licenses or ownership interest in any future Intellectual Property related to the operation or conduct of the business of Borrower or Borrower Products. Borrower has not received any written notice or claim, or, to the knowledge of Borrower, oral notice or claim, challenging or questioning Borrower's ownership in any Intellectual Property (or written notice of any claim challenging or questioning the ownership in any licensed Intellectual Property of the owner thereof) or suggesting that any third party has any claim of legal or beneficial ownership with respect thereto nor, to Borrower's knowledge, is there a reasonable basis for any such claim. To Borrower's knowledge, neither Borrower's use of its Intellectual Property nor the production and sale of Borrower Products infringes the Intellectual Property or other rights of others.

5.12 Financial Accounts. Exhibit E, as may be updated by the Borrower in a written notice provided to Lender after the Closing Date, is a true, correct and complete list of (a) all banks and other financial institutions at which Borrower or any Subsidiary maintains Deposit Accounts and (b) all institutions at which Borrower or any Subsidiary maintains an account holding Investment Property, and such exhibit correctly identifies the name, address and telephone number of each bank or other institution, the name in which the account is held, a description of the purpose of the account, and the complete account number therefor.

5.13 Employee Loans. Borrower has no outstanding loans to any employee, officer or director of the Borrower nor has Borrower guaranteed the payment of any loan made to an employee, officer or director of the Borrower by a third party.

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5.14 Capitalization and Subsidiaries. Borrower's capitalization as of the Closing Date is set forth on Schedule 5.14 annexed hereto. Borrower does not own any stock, partnership interest or other securities of any Person, except for Permitted Investments. Attached as Schedule 5.14, as may be updated by Borrower in a written notice provided after the Closing Date, is a true, correct and complete list of each Subsidiary.

5.15 Centre of main interests and establishments. Borrower has its "centre of main interests" (as that term is used in article 3(1) of The Council of the European Union Regulation No. 1346/2000 on Insolvency Proceedings) in the Netherlands.

## **SECTION 6. INSURANCE; INDEMNIFICATION**



6.1 **Coverage.** Borrower shall cause to be carried and maintained commercial general liability insurance, on an occurrence form, against risks customarily insured against in Borrower's line of business. Such risks shall include the risks of bodily injury, including death, property damage, personal injury, advertising injury, and contractual liability per the terms of the indemnification agreement found in Section 6.3. Borrower must maintain a minimum of \$1,000,000 of commercial general liability insurance for each occurrence and \$2,000,000 in the aggregate. Borrower has and agrees to maintain a minimum of \$2,000,000 of directors' and officers' insurance for each occurrence and \$5,000,000 in the aggregate. So long as there are any Secured Obligations outstanding, Borrower shall also cause to be carried and maintained insurance upon the Collateral, insuring against all risks of physical loss or damage howsoever caused, in an amount not less than the full replacement cost of the Collateral, provided that such insurance may be subject to standard exceptions and deductibles. Borrower shall also carry and maintain a fidelity insurance policy in an amount not less than \$100,000.

6.2 **Certificates.** Borrower shall deliver to Lender certificates of insurance that evidence Borrower's compliance with its insurance obligations in Section 6.1 and the obligations contained in this Section 6.2. Borrower's insurance certificate shall state Lender is an additional insured for commercial general liability, a loss payee for all risk property damage insurance, subject to the insurer's approval, a loss payee for fidelity insurance, and a loss payee for property insurance and additional insured for liability insurance for any future insurance that Borrower may acquire from such insurer. Attached to the certificates of insurance will be additional insured endorsements for liability and lender's loss payable endorsements for all risk property damage insurance and fidelity. Unless an Event of Default shall have occurred and be continuing, all insurance proceeds shall be paid or turned over to Borrower. All certificates of insurance will provide for a minimum of thirty (30) days advance written notice to Lender of cancellation or any other change adverse to Lender's interests. Any failure of Lender to scrutinize such insurance certificates for compliance is not a waiver of any of Lender's rights, all of which are reserved.

6.3 **Indemnity.** Borrower agrees to indemnify and hold Lender and its officers, directors, employees, agents, in-house attorneys, representatives and shareholders harmless from and against any and all claims, costs, expenses, damages and liabilities (including such claims, costs, expenses, damages and liabilities based on liability in tort, including strict liability in tort), including reasonable documented attorneys' fees and disbursements and other costs of investigation or defense (including those incurred upon any appeal), that may be instituted or

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asserted against or incurred by Lender or any such Person as the result of credit having been extended, suspended or terminated under this Agreement and the other Loan Documents or the administration of such credit, or in connection with or arising out of the transactions contemplated hereunder and thereunder, or any actions or failures to act in connection therewith, or arising out of the disposition or utilization of the Collateral, excluding in all cases claims resulting solely from Lender's gross negligence or willful misconduct. Borrower agrees to pay, and to save Lender harmless from, any and all liabilities with respect to, or resulting from any delay in paying, any and all excise, sales or other similar taxes (excluding taxes imposed on or measured by the net income of Lender) that may be payable or determined to be payable with respect to any of the Collateral or this Agreement.

## **SECTION 7. COVENANTS OF BORROWER AND UNIQUE HOLDINGS (THE LATTER SOLELY FOR PURPOSES OF SECTION 7.15)**

Borrower agrees as follows:

7.1 **Financial Reports.** Borrower shall furnish to Lender the financial statements and reports listed hereinafter (the "Financial Statements"):

(a) as soon as practicable (and in any event within 30 days) after the end of each month, unaudited interim and year-to-date financial statements as of the end of such month (prepared on a consolidated and consolidating basis, if applicable), including balance sheet and related statements of income and cash flows accompanied by a report detailing any material contingencies (including the commencement of any material litigation by or against Borrower) or any other occurrence that would reasonably be expected to have a Material Adverse Effect, all certified by Borrower's Chief Executive Officer or Chief Financial Officer to the effect that they have been prepared in accordance with IFRS, except (i) for the absence of footnotes, (ii) that they are subject to normal year-end adjustments, and (iii) they do not contain certain non-cash items that are customarily included in quarterly and annual financial statements;

(b) as soon as practicable (and in any event within 45 days) after the end of each calendar quarter, unaudited interim and year-to-date financial statements as of the end of such calendar quarter (prepared on a consolidated and consolidating basis, if applicable), including balance sheet and related statements of income and cash flows accompanied by a report detailing any material contingencies (including the commencement of any material litigation by or against Borrower) or any other occurrence that would reasonably be expected to have a Material Adverse Effect, certified by Borrower's Chief Executive Officer or Chief Financial Officer to the effect that they have been prepared in accordance with IFRS, except (i) for the absence of footnotes, and (ii) that they are subject to normal year-end adjustments; as well as the most recent capitalization table for Borrower, including the weighted average exercise price of employee stock options;

(c) as soon as practicable (and in any event within one hundred fifty (150) days) after the end of each fiscal year, unqualified audited financial statements as of the end of such year (prepared on a consolidated and consolidating basis, if applicable), including balance sheet and related statements of income and cash flows, and setting forth in comparative form the corresponding figures for the preceding fiscal year, certified by a firm of independent certified

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public accountants selected by Borrower and reasonably acceptable to Lender, accompanied by any management report from such accountants;

(d) as soon as practicable (and in any event within 30 days) after the end of each month, a Compliance Certificate in the form of Exhibit F;

(e) promptly after the sending or filing thereof, as the case may be, copies of any proxy statements, financial statements or reports that US Borrower has made available to holders of its capital stock and copies of any regular, periodic and special reports or registration statements that US Borrower files with the Securities and Exchange Commission or any governmental authority that may be substituted therefor, or any national securities exchange, including;

(f) notify Lender in writing at least two (2) weeks in advance of the time and place of any regularly scheduled meeting of the board of Directors of Borrower (including without limitation telephone, conference call and video meetings). Borrower shall give Lender copies of all notices, minutes, consents and other materials the Borrower provides to its directors in connection with said meetings;

(g) Borrower at all times shall maintain cash and/or cash equivalents on deposit in a deposit or security account located in the United States that is subject to an Account Control Agreement of at least the lesser of (i) 100% of the then outstanding principal amount of the Advance or (ii) 50% of all of the

worldwide cash and cash equivalents of the Borrower;

(h) financial and business projections promptly following their approval by Borrower's board of Directors, as well as budgets, operating plans and other financial information with respect to Borrower or its Subsidiaries reasonably requested by Lender;

(i) Borrower shall not make any change in its (a) accounting policies or reporting practices except in accordance with IFRS, or (b) fiscal years or fiscal quarters. The fiscal year of Borrower shall end on December 31; and

(j) The executed Compliance Certificate may be sent via facsimile to Lender at (650) 473-9194 or via e-mail to BJadot@HTGC.com. All Financial Statements required to be delivered pursuant to clauses (a), (b) and (c) shall be sent via e-mail to financialstatements@herculestech.com with a copy to BJadot@HTGC.com and BBang@HTGC.com provided, that if e-mail is not available or sending such Financial Statements via e-mail is not possible, they shall be sent via facsimile to Lender at: (866) 468-8916, attention Chief Credit Officer.

7.2 Management Rights. Borrower shall permit any representative that Lender authorizes, including its attorneys and accountants, to inspect the Collateral and examine and make copies and abstracts of the books of account and records of Borrower at reasonable times and upon reasonable notice during normal business hours. In addition, any such representative shall have the right to meet with management and officers of Borrower to discuss such books of account and records. In addition, Lender shall be entitled at reasonable times and intervals to consult with and advise the management and officers of Borrower concerning significant

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business issues affecting Borrower. Such consultations shall not unreasonably interfere with Borrower's business operations. The parties intend that the rights granted Lender shall constitute "management rights" within the meaning of 29 C.F.R Section 2510.3-101(d)(3)(ii), but that any advice, recommendations or participation by Lender with respect to any business issues shall not be deemed to give Lender, nor be deemed an exercise by Lender of, control over Borrower's management or policies.

7.3 Further Assurances. Borrower shall from time to time execute, deliver and file, alone or with Lender, any financing statements, security agreements, collateral assignments, notices, control agreements, or other documents to perfect or give the highest priority to Lender's Lien on the Collateral. Borrower shall from time to time procure any instruments or documents as may reasonably be requested by Lender, and take all further action that may be necessary or desirable, or that Lender may reasonably request, to perfect and protect the Liens granted hereby and thereby. In addition, and for such purposes only, Borrower hereby authorizes Lender to execute and deliver on behalf of Borrower and to file such financing statements, collateral assignments, notices, control agreements, security agreements and other documents necessary to grant, perfect and give the highest priority to Lender's Lien on the Collateral without the signature of Borrower either in Lender's name or in the name of Lender as agent and attorney-in-fact for Borrower. Borrower shall protect and defend Borrower's title to the Collateral and Lender's Lien thereon against all Persons claiming any interest adverse to Borrower or Lender other than Permitted Liens.

7.4 Indebtedness. Borrower shall not create, incur, assume, guarantee or be or remain liable with respect to any Indebtedness, or permit any Subsidiary so to do, other than Permitted Indebtedness, or prepay any Indebtedness or take any actions which impose on Borrower an obligation to prepay any Indebtedness, except for the conversion of Indebtedness into equity securities and the payment of cash in lieu of fractional shares in connection with such conversion.

7.5 Collateral. Borrower shall at all times keep the Collateral, the Intellectual Property and all other property and assets used in Borrower's business or in which Borrower now or hereafter holds any interest free and clear from any legal process or Liens whatsoever (except for Permitted Liens), and shall give Lender prompt written notice of any legal process affecting the Collateral, the Intellectual Property, such other property and assets, or any Liens thereon. Borrower shall cause its Subsidiaries to protect and defend such Subsidiary's title to its assets from and against all Persons claiming any interest adverse to such Subsidiary, and Borrower shall cause its Subsidiaries at all times to keep such Subsidiary's property and assets free and clear from any legal process or Liens whatsoever (except for Permitted Liens), and shall give Lender prompt written notice of any legal process affecting such Subsidiary's assets. Borrower shall not agree with any Person other than Lender not to encumber its property.

7.6 Investments. Borrower shall not directly or indirectly acquire or own, or make any Investment in or to any Person, or permit any of its Subsidiaries so to do, other than Permitted Investments.

7.7 Distributions. Borrower shall not, and shall not allow any Subsidiary to, (a) repurchase or redeem any class of stock or other equity interest other than pursuant to employee,

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director or consultant repurchase plans, stock option plans or agreements, restricted stock agreements or other similar agreements, provided, however, in each case the repurchase or redemption price does not exceed the original consideration paid for such stock or equity interest, or (b) declare or pay any cash dividend or make a cash distribution on any class of stock or other equity interest, except that a Subsidiary may pay dividends or make distributions to Borrower, or (c) lend money to any employees, officers or directors or guarantee the payment of any such loans granted by a third party in excess of \$250,000 in the aggregate or (d) waive, release or forgive any indebtedness owed by any employees, officers or directors in excess of \$250,000 in the aggregate.

7.8 Transfers. Except for Permitted Transfers, Borrower shall not voluntarily or involuntarily transfer, sell, lease, license, lend or in any other manner convey any equitable, beneficial or legal interest in any material portion of their assets.

7.9 Mergers or Acquisitions. Borrower shall not merge or consolidate, or permit any of its Subsidiaries to merge or consolidate, with or into any other business organization (other than mergers or consolidations of (i) a Subsidiary into Borrower, or (ii) of a Subsidiary which is not a Borrower into any Subsidiary or into Borrower; provided, in each case, that with respect to any merger into Borrower, Borrower is the surviving entity) or acquire, or permit any of its Subsidiaries to acquire, all or substantially all of the capital stock or property of another Person.

7.10 Taxes. Borrower and its Subsidiaries shall pay when due all taxes, fees or other charges of any nature whatsoever (together with any related interest or penalties) now or hereafter imposed or assessed against Borrower, Lender or the Collateral or upon Borrower's ownership, possession, use, operation or disposition thereof or upon Borrower's rents, receipts or earnings arising therefrom. Borrower shall file on or before the due date therefor all personal property tax returns in respect of the Collateral. Notwithstanding the foregoing, Borrower may contest, in good faith and by appropriate proceedings, taxes for which Borrower maintains adequate reserves therefor in accordance with IFRS.

7.11 Corporate Changes. Neither Borrower nor any Subsidiary shall change its corporate name, legal form or jurisdiction of formation without twenty (20) days' prior written notice to Lender. Neither Borrower nor any Subsidiary shall relocate its chief executive office or its principal place of business unless: (i) it has provided prior written notice to Lender; and (ii) such relocation shall be within the Netherlands. Neither Borrower nor any Subsidiary shall relocate any item of Collateral (other than (x) sales of movable assets in the ordinary course of business, (y) relocations of movable assets having an aggregate value of up to \$250,000 in any fiscal year, and (z) relocations of Collateral from a location described on Exhibit C to another location described on Exhibit C) unless (i) it has provided prompt written notice to Lender, (ii) such relocation is within the Netherlands and, (iii) if such relocation is to a third party bailee, it has delivered a bailee agreement in form and substance reasonably acceptable to Lender.

7.12 Deposit Accounts. Neither Borrower nor any Subsidiary shall maintain any Deposit Accounts (other than payroll, trust or escrow accounts), or accounts holding Investment Property, except with respect to which Lender has an Account Control Agreement and/or a first ranking right of pledge.

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7.13 Subsidiaries. Borrower shall notify Lender of each Subsidiary formed subsequent to the Closing Date and, within 15 days of formation, shall cause any such Subsidiary to execute and deliver to Lender a Joinder Agreement.

7.14 Pensions. Borrower shall ensure that all pension schemes operated by or maintained for the benefit of members of the group and/or any of their employees are funded to the extent required by applicable law and regulations where failure to do so would be reasonably likely to have a Material Adverse Effect.

7.15 uniQure Holdings. uniQure Holdings shall transfer the amount of € 14,000,000 (minus related costs) to be received in connection with the Chiesi transaction to a Deposit Account in the name of uniQure within 10 working days upon receipt thereof. During the period of receipt by uniQure Holdings and the actual transfer of such amount, uniQure Holdings shall in no event grant or create any Lien on such amount or any part thereof to or for the benefit of any third party.

## **SECTION 8. RIGHT TO INVEST**

8.1 Lender or its assignee or nominee shall have the right, in its discretion, to participate in any Subsequent Financing in an amount of up to Two Million Dollars (\$2,000,000) on the same terms, conditions and pricing afforded to others participating in any such Subsequent Financing.

## **SECTION 9. EVENTS OF DEFAULT**

The occurrence of any one or more of the following events shall be an Event of Default:

9.1 Payments. Borrower fails to pay any amount when due under this Agreement or any of the other Loan Documents; or

9.2 Covenants. Borrower breaches or defaults in the performance of any covenant or Secured Obligation under this Agreement, or any of the other Loan Documents, and (a) with respect to a default under any covenant under this Agreement (other than under Sections [6,] 7.1(g), 7.5, 7.6, 7.7, 7.8 or 7.9) such default continues for more than fifteen (15) days after the earlier of the date on which (i) Lender has given notice of such default to Borrower and (ii) Borrower has actual knowledge of such default or (b) with respect to a default under any of Sections [6,] 7.1(g), 7.5, 7.6, 7.7, 7.8 or 7.9, the occurrence of such default; or

9.3 Material Adverse Effect. A circumstance has occurred that would reasonably be expected to have a Material Adverse Effect; or

9.4 Other Loan Documents. The occurrence of any default under any Loan Document and such default continues for more than ten (10) days after the earlier of (a) Lender has given notice of such default to Borrower, or (b) Borrower has actual knowledge of such default; or

9.5 Representations. Any material representation or warranty made by Borrower in any Loan Document shall have been false or misleading in any material respect; or

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9.6 Insolvency. Borrower (A) (i) shall make an assignment for the benefit of creditors; or (ii) shall be unable to pay its debts as they become due, or be unable to pay or perform under the Loan Documents, or shall become insolvent; or (iii) shall file a voluntary petition in bankruptcy; or (iv) shall file any petition, answer, or document seeking for itself any reorganization, arrangement, composition, readjustment, liquidation, dissolution or similar relief under any present or future statute, law or regulation pertinent to such circumstances; or (v) shall seek or consent to or acquiesce in the appointment of any trustee, receiver, or liquidator of Borrower or of all or any substantial part (i.e., 33-1/3% or more) of the assets or property of Borrower; or (vi) shall cease operations of its business as its business has normally been conducted, or terminate substantially all of its employees; (vii) Borrower or its directors or majority shareholders shall take any action initiating any of the foregoing actions described in clauses (i) through (vi); or (B) either (i) forty-five (45) days shall have expired after the commencement of an involuntary action against Borrower seeking reorganization, arrangement, composition, readjustment, liquidation, dissolution or similar relief under any present or future statute, law or regulation, without such action being dismissed or all orders or proceedings thereunder affecting the operations or the business of Borrower being stayed; or (ii) a stay of any such order or proceedings shall thereafter be set aside and the action setting it aside shall not be timely appealed; or (iii) Borrower shall file any answer admitting or not contesting the material allegations of a petition filed against Borrower in any such proceedings; or (iv) the court in which such proceedings are pending shall enter a decree or order granting the relief sought in any such proceedings; or (v) thirty (30) days shall have expired after the appointment, without the consent or acquiescence of Borrower, of any trustee, receiver or liquidator of Borrower or of all or any substantial part of the properties of Borrower without such appointment being vacated; or

9.7 Attachments; Judgments. Any portion of Borrower's assets is attached or seized, or a levy is filed against any such assets (and such attachment, seizure or levy is not lifted or released within 30 days), or a judgment or judgments (no longer subject to appeal) is/are entered for the payment of money, individually or in the aggregate, of at least \$250,000, or Borrower is enjoined or in any way prevented by court order from conducting any part of its business; or

9.8 Other Obligations. The occurrence of any default (beyond any applicable grace, appeal or cure periods) under any agreement or obligation of Borrower involving any Indebtedness in excess of \$250,000, or the occurrence of any default by the Borrower under any agreement or obligation of Borrower that could reasonably be expected to have a Material Adverse Effect.

## SECTION 10. REMEDIES

10.1 General. Upon and during the continuance of any one or more Events of Default, (i) Lender may, at its option, accelerate and demand payment of all or any part of the Secured Obligations together with a Prepayment Charge and declare them to be immediately due and payable (provided, that upon the occurrence of an Event of Default of the type described in Section 9.6, all of the Secured Obligations shall automatically be accelerated and made due and payable, in each case without any further notice or act), and (ii) Lender may notify any of Borrower's account debtors to make payment directly to Lender, compromise the amount of any such account on Borrower's behalf and endorse Lender's name without recourse on any such payment for deposit directly to Lender's account. Lender may exercise all rights and remedies

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with respect to the Collateral under the Loan Documents or otherwise available to it under the laws of the Netherlands, the UCC and other applicable law, including the right to release, hold, sell, lease, liquidate, collect, realize upon, or otherwise dispose of all or any part of the Collateral and the right to occupy, utilize, process and commingle the Collateral.

10.2 Collection; Foreclosure. Unless otherwise agreed in the Collateral Documents, upon the occurrence and during the continuance of any Event of Default, Lender may, at any time or from time to time, apply, collect, liquidate, sell in one or more sales, lease or otherwise dispose of, any or all of the Collateral, in its then condition or following any commercially reasonable preparation or processing, in such order as Lender may elect. Any such sale may be made either at public or private sale at its place of business or elsewhere. Borrower agrees that any such public or private sale may occur upon ten (10) calendar days' prior written notice to Borrower. Lender may require Borrower to assemble the Collateral and make it available to Lender at a place designated by Lender that is reasonably convenient to Lender and Borrower. The proceeds of any sale, disposition or other realization upon all or any part of the Collateral shall be applied by Lender in the following order of priorities:

First, to Lender in an amount sufficient to pay in full Lender's costs and professionals' and advisors' fees and expenses as described in Section 11.11;

Second, to Lender in an amount equal to the then unpaid amount of the Secured Obligations (including principal, interest, and the Default Rate interest), in such order and priority as Lender may choose in its sole discretion; and

Finally, after the full, final, and indefeasible payment in Cash of all of the Secured Obligations, to any creditor holding a junior Lien on the Collateral, or to Borrower or its representatives or as a court of competent jurisdiction may direct.

Lender shall be deemed to have acted reasonably in the custody, preservation and disposition of any of the Collateral if it complies with the obligations of a secured party under the UCC.

10.3 No Waiver. Lender shall be under no obligation to marshal any of the Collateral for the benefit of Borrower or any other Person, and Borrower expressly waives all rights, if any, to require Lender to marshal any Collateral.

10.4 Cumulative Remedies. The rights, powers and remedies of Lender hereunder shall be in addition to all rights, powers and remedies given by statute or rule of law and are cumulative. The exercise of any one or more of the rights, powers and remedies provided herein shall not be construed as a waiver of or election of remedies with respect to any other rights, powers and remedies of Lender.

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## SECTION 11. MISCELLANEOUS

11.1 Severability. Whenever possible, each provision of this Agreement shall be interpreted in such manner as to be effective and valid under applicable law, but if any provision of this Agreement shall be prohibited by or invalid under such law, such provision shall be ineffective only to the extent and duration of such prohibition or invalidity, without invalidating the remainder of such provision or the remaining provisions of this Agreement.

11.2 Notice. Except as otherwise provided herein, any notice, demand, request, consent, approval, declaration, service of process or other communication (including the delivery of Financial Statements) that is required, contemplated, or permitted under the Loan Documents or with respect to the subject matter hereof shall be in writing, and shall be deemed to have been validly served, given, delivered, and received upon the earlier of: (i) the day of transmission by facsimile or hand delivery or delivery by an overnight express service or overnight mail delivery service; or (ii) the third calendar day after deposit in the United States mails, with proper first class postage prepaid, in each case addressed to the party to be notified as follows:

If to Lender:                   HERCULES TECHNOLOGY GROWTH CAPITAL, INC.  
Legal Department  
Attention: Chief Legal Officer and Mr. Bryan Jadot  
400 Hamilton Avenue, Suite 310  
Palo Alto, California 94301  
Facsimile: 650-473-9194  
Telephone: 650-289-3060

If to Borrower:               [                    ]  
Attention: [                    ]  
Facsimile:  
Tel: [                    ]

or to such other address as each party may designate for itself by like notice.

11.3 Entire Agreement; Amendments. This Agreement and the other Loan Documents constitute the entire agreement and understanding of the parties hereto in respect of the subject matter hereof and thereof, and supersede and replace in their entirety any prior proposals, term sheets, non-disclosure or confidentiality agreements, letters, negotiations or other documents or agreements, whether written or oral, with respect to the subject matter hereof or thereof

(including Lender's proposal letter dated March 1, 2013). None of the terms of this Agreement or any of the other Loan Documents may be amended except by an instrument executed by each of the parties hereto.

11.4 No Strict Construction. The parties hereto have participated jointly in the negotiation and drafting of this Agreement. In the event an ambiguity or question of intent or interpretation arises, this Agreement shall be construed as if drafted jointly by the parties hereto and no presumption or burden of proof shall arise favoring or disfavoring any party by virtue of the authorship of any provisions of this Agreement.

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11.5 No Waiver. The powers conferred upon Lender by this Agreement are solely to protect its rights hereunder and under the other Loan Documents and its interest in the Collateral and shall not impose any duty upon Lender to exercise any such powers. No omission or delay by Lender at any time to enforce any right or remedy reserved to it, or to require performance of any of the terms, covenants or provisions hereof by Borrower at any time designated, shall be a waiver of any such right or remedy to which Lender is entitled, nor shall it in any way affect the right of Lender to enforce such provisions thereafter.

11.6 Survival. All agreements, representations and warranties contained in this Agreement and the other Loan Documents or in any document delivered pursuant hereto or thereto shall be for the benefit of Lender and shall survive the execution and delivery of this Agreement and the expiration or other termination of this Agreement.

11.7 Successors and Assigns. The provisions of this Agreement and the other Loan Documents shall inure to the benefit of and be binding on Borrower and its permitted assigns (if any). Borrower shall not assign its obligations under this Agreement or any of the other Loan Documents without Lender's express prior written consent, and any such attempted assignment shall be void and of no effect. Lender may assign, transfer, or endorse its rights hereunder and under the other Loan Documents without prior notice to Borrower, and all of such rights shall inure to the benefit of Lender's successors and assigns.

11.8 Governing Law. This Agreement and the other Loan Documents shall be governed by, and construed and enforced in accordance with, the laws of the Netherlands.

11.9 Jurisdiction. The courts (*Rechtbank*) of Amsterdam, the Netherlands, subject to ordinary appeal and final appeal shall have exclusive jurisdiction to hear and determine any suit, action or proceeding and to settle any disputes arising out of or in connection with this Agreement and the other Loan Documents (including a dispute regarding the existence, validity or termination of this Agreement or the consequences of its nullity) and, for such purposes, each of the parties hereto irrevocably submits to the exclusive jurisdiction of such courts. This Section is for the benefit of the Lender only. As a result, the Lender may take proceedings relating to a dispute in any other courts with jurisdiction. To the extent allowed by law, the Lender may take concurrent proceedings in any number of jurisdictions.

11.10 Professional Fees. Borrower promises to pay Lender's documented out-of-pocket fees and expenses necessary to finalize the loan documentation, including but not limited to reasonable documented attorneys' fees, UCC searches, filing costs, and other miscellaneous expenses. In addition, Borrower promises to pay any and all reasonable documented attorneys' and other professionals' fees and expenses (including fees and expenses of in-house counsel) incurred by Lender after the Closing Date in connection with or related to: (a) the Loan; (b) the administration, collection, or enforcement of the Loan; (c) the amendment or modification of the Loan Documents; (d) any waiver, consent, release, or termination under the Loan Documents; (e) the protection, preservation, sale, lease, liquidation, or disposition of Collateral or the exercise of remedies with respect to the Collateral; (f) any legal, litigation, administrative, arbitration, or out of court proceeding in connection with or related to Borrower or the Collateral, and any appeal or review thereof; and (g) any bankruptcy, restructuring, reorganization, assignment for the benefit of creditors, workout, foreclosure, or other action related to Borrower, the Collateral, the

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Loan Documents, including representing Lender in any adversary proceeding or contested matter commenced or continued by or on behalf of Borrower's estate, and any appeal or review thereof.

11.11 Confidentiality. Lender acknowledges that all financial statements provided to Lender by Borrower and certain items of Collateral and information provided to Lender by Borrower are confidential and proprietary information of Borrower, if and to the extent such information either (x) is marked as confidential by Borrower at the time of disclosure, or (y) should reasonably be understood to be confidential (the "Confidential Information"). Accordingly, Lender agrees that any Confidential Information it may obtain in the course of acquiring, administering, or perfecting Lender's security interest in the Collateral shall not be disclosed to any other person or entity in any manner whatsoever, in whole or in part, without the prior written consent of Borrower, except that Lender may disclose any such information: (a) to its own directors, officers, employees, accountants, counsel and other professional advisors and to its affiliates if Lender in its sole discretion determines that any such party should have access to such information in connection with such party's responsibilities in connection with the Loan or this Agreement and, provided that such recipient of such Confidential Information either (i) agrees to be bound by the confidentiality provisions of this paragraph or (ii) is otherwise subject to confidentiality restrictions that reasonably protect against the disclosure of Confidential Information; (b) if such information is generally available to the public; (c) if required or appropriate in any report, statement or testimony submitted to any governmental authority having or claiming to have jurisdiction over Lender; (d) if required or appropriate in response to any summons or subpoena or in connection with any litigation, to the extent permitted or deemed advisable by Lender's counsel; (e) to comply with any legal requirement or law applicable to Lender; (f) to the extent reasonably necessary in connection with the exercise of any right or remedy under any Loan Document, including Lender's sale, lease, or other disposition of Collateral after the occurrence and during the continuance of an Event of Default; (g) to any participant or assignee of Lender or any prospective participant or assignee; provided, that such participant or assignee or prospective participant or assignee agrees in writing to be bound by this Section prior to disclosure; or (h) otherwise with the prior consent of Borrower; provided, that any disclosure made in violation of this Agreement shall not affect the obligations of Borrower or any of its affiliates or any guarantor under this Agreement or the other Loan Documents.

11.12 Assignment of Rights. Borrower acknowledges and understands that Lender may sell and assign all or part of its interest hereunder and under the Loan Documents to any person or entity (an "Assignee"). After such assignment the term "Lender" as used in the Loan Documents shall mean and include such Assignee, and such Assignee shall be vested with all rights, powers and remedies of Lender hereunder with respect to the interest so assigned; but with respect to any such interest not so transferred, Lender shall retain all rights, powers and remedies hereby given. No such assignment by Lender shall relieve Borrower of any of its obligations hereunder. Lender agrees that in the event of any transfer by it of the Note(s) (if any), it will endorse thereon a notation as to the portion of the principal of the Note(s), which shall have been paid at the time of such transfer and as to the date to which interest shall have been last paid thereon.

11.14 Revival of Secured Obligations. This Agreement and the Loan Documents shall remain in full force and effect and continue to be effective if any petition is filed by or against

Borrower for liquidation or reorganization, if Borrower becomes insolvent or makes an assignment for the benefit of creditors, if a receiver or trustee is appointed for all or any significant part of Borrower's assets, or if any payment or transfer of Collateral is recovered from Lender. The Loan Documents and the Secured Obligations and Collateral security shall continue to be effective, or shall be revived or reinstated, as the case may be, if at any time payment and performance of the Secured Obligations or any transfer of Collateral to Lender, or any part thereof is rescinded, avoided or avoidable, reduced in amount, or must otherwise be restored or returned by, or is recovered from, Lender or by any obligee of the Secured Obligations, whether as a "voidable preference," "fraudulent conveyance," or otherwise, all as though such payment, performance, or transfer of Collateral had not been made. In the event that any payment, or any part thereof, is rescinded, reduced, avoided, avoidable, restored, returned, or recovered, the Loan Documents and the Secured Obligations shall be deemed, without any further action or documentation, to have been revived and reinstated except to the extent of the full, final, and indefeasible payment to Lender in Cash.

11.13 Counterparts. This Agreement and any amendments, waivers, consents or supplements hereto may be executed in any number of counterparts, and by different parties hereto in separate counterparts, each of which when so delivered shall be deemed an original, but all of which counterparts shall constitute but one and the same instrument.

11.14 Publicity.

(a) Borrower consents to the publication and use by Lender and any of its member businesses and affiliates of (i) Borrower's name (including a brief description of the relationship between Borrower and Lender) and logo for use on Lender's website and as required for the purposes of filings with or reports to governmental authorities required by law, and (ii) after review and approval by Borrower (a) Borrower's name and a hyperlink to Borrower's web site, separately or together, in written and oral presentations, advertising, promotional and marketing materials, client lists, public relations materials or on its web site (together, the "Lender Publicity Materials"); (b) the names of officers of Borrower in the Lender Publicity Materials; and (c) Borrower's name, trademarks or servicemarks in any news release concerning Lender.

(b) Neither Borrower nor any of its member businesses and affiliates shall, without Lender's consent, publicize or use, for any purpose other than filings with or reports to governmental authorities required by law, (i) Lender's name (including a brief description of the relationship between Borrower and Lender), logo or hyperlink to Lender's web site, separately or together, in written and oral presentations, advertising, promotional and marketing materials, client lists, public relations materials or on its web site (together, the "Borrower Publicity Materials"); (ii) the names of officers of Lender in the Borrower Publicity Materials; and (iii) Lender's name, trademarks, servicemarks in any news release concerning Borrower.

(SIGNATURES TO FOLLOW)

IN WITNESS WHEREOF, Borrower and Lender have duly executed and delivered this Loan and Security Agreement as of the day and year first above written.

BORROWER:

UNIQUE BIOPHARMA B.V.

Signature: /s/ PJ Morgan

Print Name: PJ Morgan

Title: CFO

UNIQUE IP B.V.

Signature: /s/ PJ Morgan

Print Name: PJ Morgan

Title: CFO

UNIQUE B.V., solely a party hereto for purposes of Sections 2.6 and 7.15

Signature: /s/ PJ Morgan

Print Name: PJ Morgan

Title: CFO

UNIQUE RESEARCH B.V.\*

Signature: /s/ PJ Morgan

Print Name: PJ Morgan

Title: CFO

UNIQUE ASSAY DEVELOPMENT B.V.\*

Signature: /s/ PJ Morgan

Print Name: PJ Morgan

Title: CFO

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UNIQUE QA B.V.\*

Signature: /s/ PJ Morgan

Print Name: PJ Morgan

Title: CFO

UNIQUE PROCESS DEVELOPMENT B.V.\*

Signature: /s/ PJ Morgan

Print Name: PJ Morgan

Title: CFO

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\* Wholly-owned subsidiary of uniQure Biopharma B.V.

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UNIQUE MANUFACTURING B.V.\*

Signature: /s/ PJ Morgan

Print Name: PJ Morgan

Title: CFO

UNIQUE NON CLINICAL B.V.\*

Signature: /s/ PJ Morgan

Print Name: PJ Morgan

Title: CFO

UNIQUE CLINICAL B.V.\*

Signature: /s/ PJ Morgan

Print Name: PJ Morgan

Title: CFO

UNIQUE, INC.

Signature: /s/ PJ Morgan

Print Name: PJ Morgan

Title: CFO

Accepted in Palo Alto, California:

LENDER:

HERCULES TECHNOLOGY GROWTH CAPITAL, INC.

Signature: /s/Ben Bang

Print Name: Ben Bang

Title: Senior Counsel

Table of Addenda, Exhibits and Schedules

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EXHIBIT A

ADVANCE REQUEST

To: Lender: Date: , 2013

Hercules Technology Growth Capital, Inc.  
400 Hamilton Avenue, Suite 310  
Palo Alto, CA 94301  
Facsimile: 650-473-9194  
Attn:

UNIQUE BIOPHARMA B.V., a (“uniQure”), (ii) UNIQUE IP B.V., a (“uniQure IP”), (iii) each of the subsidiaries of uniQure identified on the Schedule 1 to the Agreement hereinafter referred to and the signature pages thereof (uniQure, uniQure IP and such subsidiaries are hereinafter collectively referred to as “**Borrower**”) (“Borrower”) hereby requests from Hercules Technology Growth Capital, Inc. (“Lender”) an Advance in the amount of Dollars (\$) on , (the “Advance Date”) pursuant to the Loan and Security Agreement between Borrower and Lender (the “Agreement”). Capitalized words and other terms used but not otherwise defined herein are used with the same meanings as defined in the Agreement.

Please:

(a) Issue a check payable to Borrower

or

(b) Wire Funds to Borrower's account

Bank:  
Address:

ABA Number:  
Account Number:  
Account Name:



Borrower represents that the conditions precedent to the Advance set forth in the Agreement are satisfied and shall be satisfied upon the making of such Advance, including but not limited to: (i) that no event that has had or could reasonably be expected to have a Material Adverse Effect has occurred and is continuing; (ii) that the representations and warranties set forth in the Agreement and in (if applicable) the Warrant Agreement are and shall be true and correct in all material respects on and as of the Advance Date with the same effect as though made on and as of such date, except to the extent such representations and warranties expressly relate to an earlier date; (iii) that Borrower is in compliance with all the terms and provisions set forth in each Loan Document on its part to be observed or performed; and (iv) that as of the

Advance Date, no fact or condition exists that would (or would, with the passage of time, the giving of notice, or both) constitute an Event of Default under the Loan Documents. Borrower understands and acknowledges that Lender has the right to review the financial information supporting this representation and, based upon such review in its sole discretion, Lender may decline to fund the requested Advance.

Borrower hereby represents that Borrower’s corporate status and locations have not changed since the date of the Agreement or, if the Attachment to this Advance Request is completed, are as set forth in the Attachment to this Advance Request.

Borrower agrees to notify Lender promptly before the funding of the Loan if any of the matters which have been represented above shall not be true and correct on the Borrowing Date and if Lender has received no such notice before the Advance Date then the statements set forth above shall be deemed to have been made and shall be deemed to be true and correct as of the Advance Date.

Executed as of [                    ], 2013.

BORROWER:

UNIQUE BIOPHARMA B.V.

Signature: \_\_\_\_\_

Print Name: \_\_\_\_\_

Title: \_\_\_\_\_

UNIQUE IP B.V.

Signature: \_\_\_\_\_

Print Name: \_\_\_\_\_

Title: \_\_\_\_\_

UNIQUE RESEARCH B.V.

Signature: \_\_\_\_\_

Print Name: \_\_\_\_\_

Title: \_\_\_\_\_

UNIQUE ASSAY DEVELOPMENT B.V.

Signature: \_\_\_\_\_

Print Name: \_\_\_\_\_

Title: \_\_\_\_\_

UNIQUE QA B.V.

Signature: \_\_\_\_\_

Print Name: \_\_\_\_\_

Title: \_\_\_\_\_

UNIQUE PROCESS DEVELOPMENT B.V.

Signature: \_\_\_\_\_

Print Name: \_\_\_\_\_

Title: \_\_\_\_\_

UNIQUE MANUFACTURING B.V.

Signature: \_\_\_\_\_

Print Name: \_\_\_\_\_

Title: \_\_\_\_\_

UNIQUE NON CLINICAL B.V.

Signature: \_\_\_\_\_

Print Name: \_\_\_\_\_

Title: \_\_\_\_\_

UNIQUE CLINICAL B.V.

Signature: \_\_\_\_\_

Print Name: \_\_\_\_\_

Title: \_\_\_\_\_

[US SUBSIDIARY

Signature: \_\_\_\_\_

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Print Name: \_\_\_\_\_

Title: \_\_\_\_\_ ]

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## ATTACHMENT TO ADVANCE REQUEST

Dated:

Borrower hereby represents and warrants to Lender that Borrower’s current name and organizational status is as follows:

Name:

Type of organization:

State of organization:

Organization file number:

Borrower hereby represents and warrants to Lender that the street addresses, cities, states and postal codes of its current locations are as follows:

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## EXHIBIT B

### PROMISSORY NOTE

\$[ ],000,000

Advance Date: , 20[ ]

Maturity Date: , 20[ ]

FOR VALUE RECEIVED, (i) UNIQUE BIOPHARMA B.V., a (“**uniQure**”), (ii) UNIQUE IP B.V., a (“**uniQure IP**”), (iii) each of the subsidiaries of uniQure identified on the signature page hereof (uniQure, uniQure IP and such subsidiaries are hereinafter collectively referred to as “**Borrower**”) hereby promises to pay to the order of Hercules Technology Growth Capital, Inc., a Maryland corporation or the holder of this Note (the “**Lender**”) at 400 Hamilton Avenue, Suite 310, Palo Alto, CA 94301 or such other place of payment as the holder of this Secured Term Promissory Note (this “**Promissory Note**”) may specify from time to time in writing, in lawful money of the United States of America, the principal amount of [ ] Million Dollars (\$[ ],000,000) or such other principal amount as Lender has advanced to Borrower, together with interest at a floating rate per annum equal to the greater of either (i) eleven and eighty-five one-hundredths of one percent (11.85%), or (ii) the sum of (A) eleven and eighty-five one-hundredths of one percent (11.85%), plus

(B) the Prime Rate minus three and one quarter of one percent (3.25%) based upon a year consisting of 360 days, with interest computed daily based on the actual number of days in each month.

This Promissory Note is the Note referred to in, and is executed and delivered in connection with, that certain Loan and Security Agreement dated       , 2013, by and between Borrower and Lender (as the same may from time to time be amended, modified or supplemented in accordance with its terms, the “Loan Agreement”), and is entitled to the benefit and security of the Loan Agreement and the other Loan Documents (as defined in the Loan Agreement), to which reference is made for a statement of all of the terms and conditions thereof. All payments shall be made in accordance with the Loan Agreement. All terms defined in the Loan Agreement shall have the same definitions when used herein, unless otherwise defined herein. An Event of Default under the Loan Agreement shall constitute a default under this Promissory Note.

Borrower agrees to make all payments under this Promissory Note without setoff, recoupment or deduction and regardless of any counterclaim or defense. This Promissory Note has been negotiated and delivered to Lender and is payable in the State of California. This Promissory Note shall be governed by and construed and enforced in accordance with, the laws of the Netherlands, excluding any conflicts of law rules or principles that would cause the application of the laws of any other jurisdiction.

BORROWER:

UNIQUE BIOPHARMA B.V.

Signature: \_\_\_\_\_

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Print Name: \_\_\_\_\_

Title: \_\_\_\_\_

UNIQUE IP B.V.

Signature: \_\_\_\_\_

Print Name: \_\_\_\_\_

Title: \_\_\_\_\_

UNIQUE RESEARCH B.V.

Signature: \_\_\_\_\_

Print Name: \_\_\_\_\_

Title: \_\_\_\_\_

UNIQUE ASSAY DEVELOPMENT B.V.

Signature: \_\_\_\_\_

Print Name: \_\_\_\_\_

Title: \_\_\_\_\_

UNIQUE QA B.V.

Signature: \_\_\_\_\_

Print Name: \_\_\_\_\_

Title: \_\_\_\_\_

UNIQUE PROCESS DEVELOPMENT B.V.

Signature: \_\_\_\_\_

Print Name: \_\_\_\_\_

Title: \_\_\_\_\_

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UNIQUE MANUFACTURING B.V.

Signature: \_\_\_\_\_

Print Name: \_\_\_\_\_

Title: \_\_\_\_\_

UNIQUE NON CLINICAL B.V.

Signature: \_\_\_\_\_

Print Name: \_\_\_\_\_

Title: \_\_\_\_\_

UNIQUE CLINICAL B.V.

Signature: \_\_\_\_\_

Print Name: \_\_\_\_\_

Title: \_\_\_\_\_

[US SUBSIDIARY

Signature: \_\_\_\_\_

Print Name: \_\_\_\_\_

Title: \_\_\_\_\_]

EXHIBIT C

NAME, LOCATIONS, AND OTHER INFORMATION FOR BORROWER

1. Borrower represents and warrants to Lender that Borrower’s current name and organizational status as of the Closing Date is as follows:

Name:

Type of organization:

State of organization:

Organization file number:

2. Borrower represents and warrants to Lender that for five (5) years prior to the Closing Date, Borrower did not do business under any other name or organization or form except the following:

Name:

Used during dates of:

Type of Organization:

State of organization:

Organization file Number:

Borrower’s fiscal year ends on

[US] Borrower’s federal employer tax identification number is:

3. Borrower represents and warrants to Lender that its chief executive office is located at .

EXHIBIT D

BORROWER’S PATENTS, TRADEMARKS, COPYRIGHTS AND LICENSES

Case Ref.	UniQure Ref.	Country	Owners	Title	Official No.	Case Status	Filing Date	Date Registration
P042011PCT		PCT	Amsterdam Molecular Therapeutics B.V.		PCT/NL98/00234	Abandoned	27-Apr-1998	
P044950EP/AT	84134-6(ren)	Austria	Amsterdam Molecular Therapeutics B.V.;University of British Columbia	Lipoprotein lipase (LPL) variant therapeutics	1200117	Granted	23-Jun-2000	13-Aug-2008
P044950EP/BE	84134-6(ren)	Belgium	Amsterdam Molecular Therapeutics B.V.;University of British Columbia	Lipoprotein lipase (LPL) variant therapeutics	1200117	Granted	23-Jun-2000	13-Aug-2008
P044950EP/CH	84134-6(ren)	Switzerland	Amsterdam Molecular Therapeutics B.V.;University of British Columbia	Lipoprotein lipase (LPL) variant therapeutics	1200117	Granted	23-Jun-2000	13-Aug-2008
P044950EP/CY	84134-6(ren)	Cyprus	Amsterdam Molecular	Lipoprotein lipase	1200117	Granted	23-Jun-2000	13-Aug-2008

			Therapeutics B.V.;University of British Columbia	(LPL) variant therapeutics				
P044950EP/DE	84134-6(ren)	Germany	Amsterdam Molecular Therapeutics B.V.;University of British Columbia	Lipoprotein lipase (LPL) variant therapeutics	60039880.3	Granted	23-Jun-2000	13-Aug-2008
P044950EP/DK	84134-6(ren)	Denmark	Amsterdam Molecular Therapeutics B.V.;University of British Columbia	Lipoprotein lipase (LPL) variant therapeutics	1200117	Granted	23-Jun-2000	13-Aug-2008
P044950EP/ES	84134-6(ren)	Spain	Amsterdam Molecular Therapeutics B.V.;University of British Columbia	Lipoprotein lipase (LPL) variant therapeutics	1200117	Granted	23-Jun-2000	13-Aug-2008
P044950EP/FI	84134-6(ren)	Finland	Amsterdam Molecular Therapeutics B.V.;University of British Columbia	Lipoprotein lipase (LPL) variant therapeutics	1200117	Granted	23-Jun-2000	13-Aug-2008
P044950EP/FR	84134-6(ren)	France	Amsterdam Molecular Therapeutics B.V.;University of British Columbia	Lipoprotein lipase (LPL) variant therapeutics	1200117	Granted	23-Jun-2000	13-Aug-2008
P044950EP/GB	84134-6(ren)	United Kingdom	Amsterdam Molecular Therapeutics B.V.;University of British Columbia	Lipoprotein lipase (LPL) variant therapeutics	1200117	Granted	23-Jun-2000	13-Aug-2008
P044950EP/GR	84134-6(ren)	Greece	Amsterdam Molecular Therapeutics B.V.;University of British Columbia	Lipoprotein lipase (LPL) variant therapeutics	1200117	Granted	23-Jun-2000	13-Aug-2008
P044950EP/IE	84134-6(ren)	Ireland	Amsterdam Molecular Therapeutics	Lipoprotein lipase (LPL) variant	1200117	Granted	23-Jun-2000	13-Aug-2008

Case Ref.	UniQure Ref.	Country	Owners	Title	Official No.	Case Status	Filing Date	Date Registration
P044950EP/IT	84134-6(ren)	Italy	B.V.;University of British Columbia Amsterdam Molecular Therapeutics B.V.;University of British Columbia	therapeutics Lipoprotein lipase (LPL) variant therapeutics	1200117	Granted	23-Jun-2000	13-Aug-2008
P044950EP/LU	84134-6(ren)	Luxembourg	Amsterdam Molecular Therapeutics B.V.;University of British Columbia	Lipoprotein lipase (LPL) variant therapeutics	1200117	Granted	23-Jun-2000	13-Aug-2008
P044950EP/MC	84134-6(ren)	Monaco	Amsterdam Molecular Therapeutics B.V.;University of British Columbia	Lipoprotein lipase (LPL) variant therapeutics	1200117	Granted	23-Jun-2000	13-Aug-2008
P044950EP/NL	84134-6(ren)	The Netherlands	Amsterdam Molecular Therapeutics B.V.;University of British Columbia	Lipoprotein lipase (LPL) variant therapeutics	1200117	Granted	23-Jun-2000	13-Aug-2008
P044950EP/PT	84134-6(ren)	Portugal	Amsterdam Molecular Therapeutics B.V.;University of British Columbia	Lipoprotein lipase (LPL) variant therapeutics	1200117	Granted	23-Jun-2000	13-Aug-2008
P044950EP/SE	84134-6(ren)	Sweden	Amsterdam Molecular Therapeutics B.V.;University of British Columbia	Lipoprotein lipase (LPL) variant therapeutics	1200117	Granted	23-Jun-2000	13-Aug-2008
P044950PCT/EP	84134-4	Europe	Amsterdam Molecular Therapeutics B.V.;University of British Columbia	Lipoprotein lipase (LPL) variant therapeutics	1200117	Granted	23-Jun-2000	13-Aug-2008
P044976EP	AMT-P102	Europe	Academisch Ziekenhuis bij de Universiteit van Amsterdam	IL-10 gene transfer to peripheral mononuclear cells	02075895.9	Abandoned	07-Mar-2002	
P044976PCT	00062 WO	PCT	Academisch Ziekenhuis bij de Universiteit van Amsterdam	IL-10 gene transfer to peripheral mononuclear cells	PCT/NL03/00170	converted	07-Mar-2003	
P044976PCT/EP	AMT-P102	Europe	Amsterdam Molecular Therapeutics (AMT) B.V.	IL-10 gene transfer to peripheral mononuclear cells	1481054	Published	07-Mar-2003	
P044976PCT/US	AMT-P102	United States of America	Amsterdam Molecular Therapeutics (AMT) IP B.V.	IL-10 gene transfer to peripheral mononuclear cells	8,119,401	Granted	07-Mar-2003	21-Feb-2012
P212752PCT		PCT	Academisch Ziekenhuis bij de Universiteit van Amsterdam	Polymorphisms in the dihydropyriminidase gene	PCT/NL03/00936	Abandoned	24-Dec-2003	
P215797EP/AT	AMT-P103	Austria	Amsterdam Molecular Therapeutics B.V.	Treatment of non alcoholic steatotic hepatitis (NASH)	1761273	Granted	20-Jun-2005	14-May-2008

Case Ref.	UniQure Ref.	Country	Owners	Title	Official No.	Case Status	Filing Date	Date Registration
P215797EP/BE	AMT-P103	Belgium	Amsterdam Molecular Therapeutics B.V.	Treatment of non alcoholic steatotic hepatitis (NASH)	1761273	Granted	20-Jun-2005	14-May-2008
P215797EP/BG	AMT-P103	Bulgaria	Amsterdam Molecular Therapeutics B.V.	Treatment of non alcoholic steatotic hepatitis (NASH)	1761273	Abandoned	20-Jun-2005	14-May-2008
P215797EP/CH	AMT-P103	Switzerland	Amsterdam Molecular Therapeutics B.V.	Treatment of non alcoholic steatotic hepatitis (NASH)	1761273	Granted	20-Jun-2005	14-May-2008
P215797EP/CY	AMT-P103	Cyprus	Amsterdam Molecular Therapeutics B.V.	Treatment of non alcoholic steatotic hepatitis (NASH)	1761273	Abandoned	20-Jun-2005	14-May-2008
P215797EP/CZ	AMT-P103	Czech Republic	Amsterdam Molecular Therapeutics B.V.	Treatment of non alcoholic steatotic hepatitis (NASH)	1761273	Abandoned	20-Jun-2005	14-May-2008
P215797EP/DE	AMT-P103	Germany	Amsterdam Molecular Therapeutics B.V.	Treatment of non alcoholic steatotic hepatitis (NASH)	1761273	Granted	20-Jun-2005	14-May-2008
P215797EP/DK	AMT-P103	Denmark	Amsterdam Molecular	Treatment of non	1761273	Granted	20-Jun-2005	14-May-2008

			Therapeutics B.V.	alcoholic steatotic hepatitis (NASH)				
P215797EP/EE	AMT-P103	Estonia	Amsterdam Molecular Therapeutics B.V.	Treatment of non alcoholic steatotic hepatitis (NASH)	1761273	Abandoned	20-Jun-2005	14-May-2008
P215797EP/ES	AMT-P103	Spain	Amsterdam Molecular Therapeutics B.V.	Treatment of non alcoholic steatotic hepatitis (NASH)	1761273	Granted	20-Jun-2005	14-May-2008
P215797EP/FI	AMT-P103	Finland	Amsterdam Molecular Therapeutics B.V.	Treatment of non alcoholic steatotic hepatitis (NASH)	1761273	Abandoned	20-Jun-2005	14-May-2008
P215797EP/FR	AMT-P103	France	Amsterdam Molecular Therapeutics B.V.	Treatment of non alcoholic steatotic hepatitis (NASH)	1761273	Granted	20-Jun-2005	14-May-2008
P215797EP/GB	AMT-P103	United Kingdom	Amsterdam Molecular Therapeutics B.V.	Treatment of non alcoholic steatotic hepatitis (NASH)	1761273	Granted	20-Jun-2005	14-May-2008
P215797EP/GR	AMT-P103	Greece	Amsterdam Molecular Therapeutics B.V.	Treatment of non alcoholic steatotic hepatitis (NASH)	1761273	Abandoned	20-Jun-2005	14-May-2008
P215797EP/HU	AMT-P103	Hungary	Amsterdam Molecular Therapeutics B.V.	Treatment of non alcoholic steatotic hepatitis (NASH)	1761273	Abandoned	20-Jun-2005	14-May-2008
P215797EP/IE	AMT-P103	Ireland	Amsterdam Molecular Therapeutics B.V.	Treatment of non alcoholic steatotic hepatitis (NASH)	1761273	Abandoned	20-Jun-2005	14-May-2008
P215797EP/IS	AMT-P103	Iceland	Amsterdam Molecular Therapeutics B.V.	Treatment of non alcoholic steatotic hepatitis (NASH)	1761273	Abandoned	20-Jun-2005	14-May-2008

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P215797EP/IT	AMT-P103	Italy	Amsterdam Molecular Therapeutics B.V.	Treatment of non alcoholic steatotic hepatitis (NASH)	1761273	Granted	20-Jun-2005	14-May-2008
P215797EP/LT	AMT-P103	Lithuania	Amsterdam Molecular Therapeutics B.V.	Treatment of non alcoholic steatotic hepatitis (NASH)	1761273	Abandoned	20-Jun-2005	14-May-2008
P215797EP/LU	AMT-P103	Luxembourg	Amsterdam Molecular Therapeutics B.V.	Treatment of non alcoholic steatotic hepatitis (NASH)	1761273	Granted	20-Jun-2005	14-May-2008
P215797EP/MC	AMT-P103	Monaco	Amsterdam Molecular Therapeutics B.V.	Treatment of non alcoholic steatotic hepatitis (NASH)	1761273	Abandoned	20-Jun-2005	14-May-2008
P215797EP/NL	AMT-P103	The Netherlands	Amsterdam Molecular Therapeutics B.V.	Treatment of non alcoholic steatotic hepatitis (NASH)	1761273	Granted	20-Jun-2005	14-May-2008
P215797EP/PL	AMT-P103	Poland	Amsterdam Molecular Therapeutics B.V.	Treatment of non alcoholic steatotic hepatitis (NASH)	1761273	Abandoned	20-Jun-2005	14-May-2008
P215797EP/PT	AMT-P103	Portugal	Amsterdam Molecular Therapeutics B.V.	Treatment of non alcoholic steatotic hepatitis (NASH)	1761273	Abandoned	20-Jun-2005	14-May-2008
P215797EP/RO	AMT-P103	Romania	Amsterdam Molecular Therapeutics B.V.	Treatment of non alcoholic steatotic hepatitis (NASH)	1761273	Abandoned	20-Jun-2005	14-May-2008
P215797EP/SE	AMT-P103	Sweden	Amsterdam Molecular Therapeutics B.V.	Treatment of non alcoholic steatotic hepatitis (NASH)	1761273	Granted	20-Jun-2005	14-May-2008
P215797EP/SI	AMT-P103	Slovenia	Amsterdam Molecular Therapeutics B.V.	Treatment of non alcoholic steatotic hepatitis (NASH)	1761273	Abandoned	20-Jun-2005	14-May-2008
P215797EP/SK	AMT-P103	Slovakia	Amsterdam Molecular Therapeutics B.V.	Treatment of non alcoholic steatotic hepatitis (NASH)	1761273	Abandoned	20-Jun-2005	14-May-2008
P215797EP/TR	AMT-P103	Turkey	Amsterdam Molecular Therapeutics B.V.	Treatment of non alcoholic steatotic hepatitis (NASH)	1761273	Granted	20-Jun-2005	14-May-2008
P215797PCT	AMT-P103	PCT	Academisch Ziekenhuis bij de Universiteit van Amsterdam;Amsterdam Molecular Therapeutics B.V.	Treatment of non alcoholic steatotic hepatitis (NASH)	PCT/NL2005/000446	Converted	20-Jun-2005	
P215797PCT/AU	AMT-P103	Australia	Amsterdam Molecular Therapeutics (AMT) B.V.	Treatment of non alcoholic steatotic hepatitis (NASH)	2005253897	Granted	20-Jun-2005	15-Dec-2011
P215797PCT/CA	AMT-P103	Canada	Academisch Ziekenhuis bij de Universiteit van Amsterdam;Amsterdam Molecular Therapeutics	Treatment of non alcoholic steatotic hepatitis (NASH)	2,568,643	Abandoned	20-Jun-2005	

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P215797PCT/CN	AMT-P103	China	B.V. Amsterdam Molecular Therapeutics B.V.	Treatment of non alcoholic steatotic hepatitis (NASH)	CN 1972709 A	Abandoned	20-Jun-2005	
P215797PCT/EP	AMT-P103	Europe	Amsterdam Molecular Therapeutics B.V.	Treatment of non alcoholic steatotic hepatitis (NASH)	1761273	Granted	20-Jun-2005	14-May-2008
P215797PCT/IN	AMT-P103	India	Academisch Ziekenhuis bij de Universiteit van Amsterdam;Amsterdam Molecular Therapeutics B.V.	Treatment of non alcoholic steatotic hepatitis (NASH)	7309/DELNP/2006	Abandoned	20-Jun-2005	
P215797PCT/JP	AMT-P103	Japan	Academisch Ziekenhuis bij de Universiteit van Amsterdam;Amsterdam Molecular Therapeutics B.V.	Treatment of non alcoholic steatotic hepatitis (NASH)	2008-503569	Abandoned	20-Jun-2005	
P215797PCT/KR	AMT-P103	Republic of Korea	Academisch Ziekenhuis bij de Universiteit van Amsterdam;Amsterdam Molecular Therapeutics B.V.	Treatment of non alcoholic steatotic hepatitis (NASH)	10-2006-7027008	Abandoned	20-Jun-2005	
P215797PCT/US	AMT-P103	United States of America	Amsterdam Molecular Therapeutics B.V.	Treatment of non alcoholic steatotic	US-2008-0280823	Abandoned	20-Jun-2005	

P215797US	AMT-P103	United States of America	Academisch Ziekenhuis bij de Universiteit van Amsterdam	hepatitis (NASH) New method of treatment for non alcoholic steatotic hepatitis (NASH)	60/580,903	Abandoned	21-Jun-2004	
P215797US1	AMT-P103WOUScont	United States of America	Amsterdam Molecular Therapeutics B.V.	Treatment of non alcoholic steatotic hepatitis (NASH)	US-2011-0081332	Abandoned	03-Dec-2010	
P6002782EP		Europe	Amsterdam Molecular Therapeutics B.V.	Methods and means for isolation of Adeno-associated virus using single chain antibodies		Abandoned		
P6004974EP/AT	AMT-P104	Austria	uniQure IP B.V.	Improved AAV vectors produced in insect cells	1945779	Not Yet Filed	19-Oct-2006	06-Mar-2013
P6004974EP/BE	AMT-P104	Belgium	uniQure IP B.V.	Improved AAV vectors produced in insect cells	1945779	Not Yet Filed	19-Oct-2006	06-Mar-2013
P6004974EP/CH	AMT-P104	Switzerland	uniQure IP B.V.	Improved AAV vectors produced in insect cells	1945779	Not Yet Filed	19-Oct-2006	06-Mar-2013
P6004974EP/DE	AMT-P104	Germany	uniQure IP B.V.	Improved AAV vectors produced in insect cells	60 2006 034 943.2	Not Yet Filed	19-Oct-2006	06-Mar-2013
P6004974EP/DK	AMT-P104	Denmark	uniQure IP B.V.	Improved AAV vectors produced in insect cells	1945779	Not Yet Filed	19-Oct-2006	06-Mar-2013
P6004974EP/ES	AMT-P104	Spain	uniQure IP B.V.	Improved AAV vectors produced in insect cells	1945779	Not Yet Filed	19-Oct-2006	06-Mar-2013

Case Ref.	UniQure Ref.	Country	Owners	Title	Official No.	Case Status	Filing Date	Date Registration
P6004974EP/FI	AMT-P104	Finland	uniQure IP B.V.	Improved AAV vectors produced in insect cells	1945779	Not Yet Filed	19-Oct-2006	06-Mar-2013
P6004974EP/FR	AMT-P104	France	uniQure IP B.V.	Improved AAV vectors produced in insect cells	1945779	Not Yet Filed	19-Oct-2006	06-Mar-2013
P6004974EP/GB	AMT-P104	United Kingdom	uniQure IP B.V.	Improved AAV vectors produced in insect cells	1945779	Not Yet Filed	19-Oct-2006	06-Mar-2013
P6004974EP/IE	AMT-P104	Ireland	uniQure IP B.V.	Improved AAV vectors produced in insect cells	1945779	Not Yet Filed	19-Oct-2006	06-Mar-2013
P6004974EP/IT	AMT-P104	Italy	uniQure IP B.V.	Improved AAV vectors produced in insect cells	1945779	Not Yet Filed	19-Oct-2006	06-Mar-2013
P6004974EP/LU	AMT-P104	Luxembourg	uniQure IP B.V.	Improved AAV vectors produced in insect cells	1945779	Not Yet Filed	19-Oct-2006	06-Mar-2013
P6004974EP/MC	AMT-P104	Monaco	uniQure IP B.V.	Improved AAV vectors produced in insect cells	1945779	Not Yet Filed	19-Oct-2006	06-Mar-2013
P6004974EP/NL	AMT-P104	The Netherlands	uniQure IP B.V.	Improved AAV vectors produced in insect cells	1945779	Granted	19-Oct-2006	06-Mar-2013
P6004974EP/SE	AMT-P104	Sweden	uniQure IP B.V.	Improved AAV vectors produced in insect cells	1945779	Not Yet Filed	19-Oct-2006	06-Mar-2013
P6004974EP/TR	AMT-P104	Turkey	uniQure IP B.V.	Improved AAV vectors produced in insect cells	1945779	Not Yet Filed	19-Oct-2006	06-Mar-2013
P6004974EP1	AMT-P104-1	Europe	uniQure IP B.V.	Improved AAV vectors produced in insect cells	2311966	Abandoned	19-Oct-2006	
P6004974EP2	AMT-P104-2	Europe	uniQure IP B.V.	Improved AAV vectors produced in insect cells	2311967	Pending	19-Oct-2006	
P6004974HK1	AMT-P104	Hong Kong	Amsterdam Molecular Therapeutics B.V.	Improved AAV vectors produced in insect cells	08111236.3	Pending	19-Oct-2006	
P6004974PCT	AMT-P104	PCT	Amsterdam Molecular Therapeutics B.V.	Improved AAV vectors produced in insect cells	PCT/NL2005/050018	Abandoned	20-Oct-2005	
P6004974PCT1	AMT-P104	PCT	Amsterdam Molecular Therapeutics B.V.	Improved AAV vectors produced in insect cells	PCT/NL2006/050262	Converted	19-Oct-2006	
P6004974PCT1/AU	AMT-P104	Australia	Amsterdam Molecular Therapeutics (AMT) IP B.V.	Improved AAV vectors produced in insect cells	2006304997	Granted	19-Oct-2006	14-Jun-2012
P6004974PCT1/CA	AMT-P104	Canada	Amsterdam Molecular Therapeutics B.V.	Improved AAV vectors produced in insect cells	2,622,233	Examination Requested	19-Oct-2006	
P6004974PCT1/CN	AMT-P104	China	Amsterdam Molecular Therapeutics B.V.	Improved AAV vectors produced in insect cells	CN 101287837 A	Published	19-Oct-2006	
P6004974PCT1/EP	AMT-P104	Europe	uniQure IP B.V.	Improved AAV vectors produced in insect cells	1945779	Granted	19-Oct-2006	06-Mar-2013
P6004974PCT1/IN	AMT-P104	India	Amsterdam Molecular Therapeutics B.V.	Improved AAV vectors produced in insect cells	2923/DELNP/2008	Examination Requested	19-Oct-2006	
P6004974PCT1/JP	AMT-P104	Japan	Amsterdam Molecular Therapeutics B.V.	Improved AAV vectors produced in insect cells	2009-512436	Examination Requested	19-Oct-2006	
P6004974PCT1/US	AMT-P104	United States of America	Amsterdam Molecular Therapeutics B.V.	Improved AAV vectors produced in insect cells	8,163,543	Granted	19-Oct-2006	24-Apr-2012
P6009362EP	AMT-P105	Europe	Amsterdam Molecular Therapeutics B.V.	AAV vectors with improved Rep coding sequences for	06115804.4	Withdraw Before Publication	21-Jun-2006	

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P6009362HK	AMT-P105	Hong Kong	Amsterdam Molecular Therapeutics B.V.	production in insect cells Vectors with modified initiation codon for the translation of AAV-REP78 useful for production of AAV in insect cells	1127083A	Pending	20-Jun-2007	
P6009362PCT	AMT-P105	PCT	Amsterdam Molecular Therapeutics B.V.	Vectors with modified initiation codon for the translation of AAV-REP78 useful for production of AAV in insect cells	PCT/NL2007/050298	converted	20-Jun-2007	
P6009362PCT/AU	AMT-P105	Australia	Amsterdam Molecular Therapeutics (AMT) IP B.V.	Vectors with modified initiation codon for the translation of AAV-REP78 useful for production of AAV in insect cells	2007261806	Examination Requested	20-Jun-2007	
P6009362PCT/CA	AMT-P105	Canada	Amsterdam Molecular Therapeutics B.V.	Vectors with modified initiation codon for the translation of AAV-REP78 useful for production of AAV in insect cells	2,655,957	Examination Requested	20-Jun-2007	
P6009362PCT/CN	AMT-P105	China	Amsterdam Molecular Therapeutics B.V.	Vectors with modified initiation codon for the translation of AAV-	CN 101506369A	Published	20-Jun-2007	

P6009362PCT/EP	AMT-P105WOEP	Europe	uniQure IP B.V.	REP78 useful for production of AAV in insect cells Vectors with modified initiation codon for the translation of AAV-REP78 useful for production of AAV in insect cells	07747521.8	Application Filed	20-Jun-2007
P6009362PCT/IL	AMT-P105	Israel	Amsterdam Molecular Therapeutics B.V.	Vectors with modified initiation codon for the translation of AAV-REP78 useful for production of AAV in insect cells	196091	Pending	20-Jun-2007
P6009362PCT/IN	AMT-P105	India	Amsterdam Molecular Therapeutics B.V.	Vectors with modified initiation codon for the translation of AAV-REP78 useful for	99/CHENP/2009	Examination Requested	20-Jun-2007

Case Ref.	UniQure Ref.	Country	Owners	Title	Official No.	Case Status	Filing Date	Date Registration
P6009362PCT/JP	AMT-P105	Japan	Amsterdam Molecular Therapeutics B.V.	production of AAV in insect cells Vectors with modified initiation codon for the translation of AAV-REP78 useful for production of AAV in insect cells	2009-540823	Examination Requested	20-Jun-2007	
P6009362PCT/KR	AMT-P105	Republic of Korea	Amsterdam Molecular Therapeutics B.V.	Vectors with modified initiation codon for the translation of AAV-REP78 useful for production of AAV in insect cells	2008-7031187	Examination Requested	20-Jun-2007	
P6009362PCT/RU	AMT-P105	Russian Federation	Amsterdam Molecular Therapeutics (AMT) IP B.V.	Vectors with modified initiation codon for the translation of AAV-REP78 useful for production of AAV in insect cells	2457252	Granted	20-Jun-2007	27-Jul-2012
P6009362PCT/US	AMT-P105	United States of America	Amsterdam Molecular Therapeutics B.V.	Vectors with modified initiation codon for the translation of AAV-REP78 useful for production of AAV in insect cells	US-2009-0191588-A1	Pending	20-Jun-2007	
P6009362US	AMT-P105	United States of America	Amsterdam Molecular Therapeutics B.V.	AAV vectors with improved Rep coding sequences for production in insect cells	60/815,262	Abandoned	21-Jun-2006	
P6016625EP	AMT-P106	Europe	Amsterdam Molecular Therapeutics B.V.	Use of AAV replication machinery for improved protein production	07075817.2	Abandoned	19-Sep-2007	
P6016625PCT	AMT-P106	PCT	Amsterdam Molecular Therapeutics B.V.	Use of AAV replication machinery for improved protein production	PCT/NL2008/050613	Converted	18-Sep-2008	
P6016625PCT/EP	AMT-P106	Europe	uniQure IP B.V.	Use of AAV replication machinery for improved protein production	2195439	Notice of 71(3) Received	18-Sep-2008	
P6016625PCT/JP	AMT-P106	Japan	Amsterdam Molecular Therapeutics B.V.	Use of AAV replication machinery for improved protein production	2010-538675	Examination Requested	18-Sep-2008	
P6016625PCT/US	AMT-P106	United States of America	Amsterdam Molecular Therapeutics (AMT) IP B.V.	Use of AAV replication machinery for improved protein production	US-2011-0119777-A1	Pending	18-Sep-2008	
P6016625US	AMT-P106	United States of America	Amsterdam Molecular Therapeutics B.V.	Use of AAV replication machinery for improved	60/973,517	Abandoned	19-Sep-2007	

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P6016639EP	AMT-P107	Europe	Amsterdam Molecular Therapeutics B.V.	protein production Baculoviral vectors comprising repeated coding sequences with differential codon biases	07113257.5	Withdraw Before Publication	26-Jul-2007	
P6016639HK	AMT-P107	Hong Kong	Amsterdam Molecular Therapeutics B.V.	Baculoviral vectors comprising repeated coding sequences with differential codon biases	1143185A	Pending	14-Oct-2010	
P6016639PCT	AMT-P107	PCT	Amsterdam Molecular Therapeutics B.V.	Baculoviral vectors comprising repeated coding sequences with differential codon biases	PCT/NL2008/050512	converted	25-Jul-2008	
P6016639PCT/AU	AMT-P107	Australia	Amsterdam Molecular Therapeutics B.V.	Baculoviral vectors comprising repeated coding sequences with differential codon biases	2008279883	Examination Requested	25-Jul-2008	
P6016639PCT/BR	AMT-P107	Brazil	Amsterdam Molecular Therapeutics B.V.	Baculoviral vectors comprising repeated coding sequences with differential codon biases	PI 0814459-1	Examination Requested	25-Jul-2008	
P6016639PCT/CA	AMT-P107	Canada	Amsterdam Molecular Therapeutics B.V.	Baculoviral vectors comprising repeated coding sequences with differential codon biases	2,694,406	Pending	25-Jul-2008	
P6016639PCT/CN	AMT-P107	China	Amsterdam Molecular Therapeutics B.V.	Baculoviral vectors comprising repeated coding sequences with	CN 101868547 A	Published	25-Jul-2008	



P6016639PCT/EA	AMT-P107	Eurasian Patent Organization	Amsterdam Molecular Therapeutics B.V.	differential codon biases Baculoviral vectors comprising repeated coding sequences with differential codon biases	201070184	Pending	25-Jul-2008
P6016639PCT/EP	AMT-P107	Europe	uniQure IP B.V.	Baculoviral vectors comprising repeated coding sequences with differential codon biases	08779058.0	Application Filed	25-Jul-2008
P6016639PCT/IL	AMT-P107	Israel	Amsterdam Molecular Therapeutics B.V.	Baculoviral vectors comprising repeated coding sequences with differential codon biases	203535	Pending	25-Jul-2008
P6016639PCT/IN	AMT-P107	India	Amsterdam Molecular Therapeutics B.V.	Baculoviral vectors comprising repeated coding sequences with differential codon biases	721/CHENP/2010	Examination Requested	25-Jul-2008
P6016639PCT/JP	AMT-P107	Japan	Amsterdam Molecular Therapeutics B.V.	Baculoviral vectors comprising repeated coding sequences with	2010-518140	Examination Requested	25-Jul-2008

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P6016639PCT/KR	AMT-P107	Republic of Korea	Amsterdam Molecular Therapeutics B.V.	differential codon biases Baculoviral vectors comprising repeated coding sequences with differential codon biases	2010-7003963	Pending	25-Jul-2008	
P6016639PCT/MX	AMT-P107	Mexico	Amsterdam Molecular Therapeutics (AMT) B.V.	Baculoviral vectors comprising repeated coding sequences with differential codon biases	294342	Granted	25-Jul-2008	05-Jan-2012
P6016639PCT/NZ	AMT-P107	New Zealand	Amsterdam Molecular Therapeutics (AMT) IP B.V.	Baculoviral vectors comprising repeated coding sequences with differential codon biases	582881	Granted	25-Jul-2008	06-Aug-2012
P6016639PCT/US	AMT-P107	United States of America	Amsterdam Molecular Therapeutics (AMT) IP B.V.	Baculoviral vectors comprising repeated coding sequences with differential codon biases	US-2010-0261254-A1	Pending	25-Jul-2008	
P6016639PCT/ZA	AMT-P107	South Africa	Amsterdam Molecular Therapeutics B.V.	Baculoviral vectors comprising repeated coding sequences with differential codon biases	2010/00561	Granted	25-Jul-2008	24-Nov-2010
P6016639US	AMT-P107	United States of America	Amsterdam Molecular Therapeutics B.V.	Baculoviral vectors comprising repeated coding sequences with differential codon biases	60/952,081	Abandoned	26-Jul-2007	
P6019170EP	AMT-P108	Europe	Amsterdam Molecular Therapeutics B.V.	Optimisation of expression of parvoviral rep and cap proteins in insect cells	08151634.6	Withdraw Before Publication	19-Feb-2008	
P6019170HK	AMT-P108	Hong Kong	Amsterdam Molecular Therapeutics (AMT) B.V.	Optimisation of expression of parvoviral rep and cap proteins in insect cells	1150630A	Published	19-Feb-2009	
P6019170PCT	AMT-P108	PCT	Amsterdam Molecular Therapeutics (AMT) B.V.	Optimisation of expression of parvoviral rep and cap proteins in insect cells	PCT/NL2009/050076	converted	19-Feb-2009	
P6019170PCT/AU	AMT-P108	Australia	Amsterdam Molecular Therapeutics (AMT) B.V.	Optimisation of expression of parvoviral rep and cap proteins in insect cells	2009215987	Examination Requested	19-Feb-2009	
P6019170PCT/CA	AMT-P108	Canada	Amsterdam Molecular Therapeutics (AMT) B.V.	Optimisation of expression of parvoviral rep and cap proteins in insect cells	2,715,924	Pending	19-Feb-2009	
P6019170PCT/CN	AMT-P108	China	Amsterdam Molecular Therapeutics (AMT) B.V.	Optimisation of expression of parvoviral rep and cap proteins in	CN 102007209 A	Published	19-Feb-2009	

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P6019170PCT/EA	AMT-P108	Eurasian Patent Organization	Amsterdam Molecular Therapeutics (AMT) B.V.	insect cells Optimisation of expression of parvoviral rep and cap proteins in insect cells	201070970	Pending	19-Feb-2009	
P6019170PCT/EP	AMT-P108	Europe	uniQure IP B.V.	Optimisation of expression of parvoviral rep and cap proteins in insect cells	09713345.8	Application Filed	19-Feb-2009	
P6019170PCT/IL	AMT-P108	Israel	Amsterdam Molecular Therapeutics (AMT) B.V.	Optimisation of expression of parvoviral rep and cap proteins in insect cells	207671	Pending	19-Feb-2009	
P6019170PCT/IN	AMT-P108	India	Amsterdam Molecular Therapeutics (AMT) B.V.	Optimisation of expression of parvoviral rep and cap proteins in insect cells	5340/CHENP/2010	Examination Requested	19-Feb-2009	
P6019170PCT/JP	AMT-P108	Japan	Amsterdam Molecular Therapeutics (AMT) B.V.	Optimisation of expression of parvoviral rep and cap proteins in insect cells	2010-547582	Examination Requested	19-Feb-2009	

P6019170PCT/US	AMT-P108	United States of America	Amsterdam Molecular Therapeutics (AMT) B.V.	Optimisation of expression of parvoviral rep and cap proteins in insect cells	US-2011-0136227-A1	Pending	19-Feb-2009
P6019170US	AMT-P107	United States of America	Amsterdam Molecular Therapeutics B.V.	Optimisation of expression of parvoviral rep and cap proteins in insect cells	61/029,673	Abandoned	19-Feb-2008
P6019299EP	AMT-P109	Europe	Amsterdam Molecular Therapeutics B.V.	Parvoviral capsid with incorporated Gly-Ala repeat region	08158418.7	Withdraw Before Publication	17-Jun-2008
P6019299PCT	AMT-P109	PCT	Amsterdam Molecular Therapeutics (AMT) B.V.	Parvoviral capsid with incorporated Gly-Ala repeat region	PCT/NL2009/050352	converted	17-Jun-2009
P6019299PCT/EP	AMT-P109	Europe	uniQure IP B.V.	Parvoviral capsid with incorporated Gly-Ala repeat region	2297185	Abandoned	17-Jun-2009
P6019299PCT/US	AMT-P109	United States of America	Amsterdam Molecular Therapeutics (AMT) B.V.	Parvoviral capsid with incorporated Gly-Ala repeat region	US-2011-0171262-A1	Abandoned	17-Jun-2009
P6019299US	AMT-P109	United States of America	Amsterdam Molecular Therapeutics B.V.	Parvoviral capsid with incorporated Gly-Ala repeat region	61/073,295	Abandoned	17-Jun-2008
P6019299US1	AMT-P109	United States of America	Amsterdam Molecular Therapeutics B.V.	Parvoviral capsid with incorporated Gly-Ala repeat region	61/073,587	Abandoned	18-Jun-2008
P6021400EP	AMT-P110	Europe	Amsterdam Molecular	Porphobilinogen	08165393.3	Withdraw	29-Sep-2008



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			Therapeutics B.V.;Proyecto de Biomedicina CIMA S.L.	deaminase gene therapy		Before Publication		
P6021400PCT	AMT-P110	PCT	Amsterdam Molecular Therapeutics (AMT) B.V.;Proyecto de Biomedicina CIMA S.L.	Porphobilinogen deaminase gene therapy	PCT/NL2009/050584	converted	29-Sep-2009	
P6021400PCT/AU	AMT-P110	Australia	Amsterdam Molecular Therapeutics (AMT) B.V.;Proyecto de Biomedicina CIMA S.L.	Porphobilinogen deaminase gene therapy	2009297243	Pending	29-Sep-2009	
P6021400PCT/BR	AMT-P110	Brazil	Amsterdam Molecular Therapeutics (AMT) B.V.;Proyecto de Biomedicina CIMA S.L.	Porphobilinogen deaminase gene therapy	PI 0919130-5	Abandoned	29-Sep-2009	
P6021400PCT/CA	AMT-P110	Canada	Amsterdam Molecular Therapeutics (AMT) B.V.;Proyecto de Biomedicina CIMA S.L.	Porphobilinogen deaminase gene therapy	2,738,969	Pending	29-Sep-2009	
P6021400PCT/CN	AMT-P110	China	Amsterdam Molecular Therapeutics (AMT) B.V.;Proyecto de Biomedicina CIMA S.L.	Porphobilinogen deaminase gene therapy	ZL 200980147755.1	Published	29-Sep-2009	
P6021400PCT/EA	AMT-P110	Eurasian Patent Organization	Amsterdam Molecular Therapeutics (AMT) B.V.;Proyecto de Biomedicina CIMA S.L.	Porphobilinogen deaminase gene therapy	201170506	Pending	29-Sep-2009	
P6021400PCT/EP	AMT-P110	Europe	uniQure IP B.V.;Proyecto de Biomedicina CIMA S.L.	Porphobilinogen deaminase gene therapy	2352823	Application Filed	29-Sep-2009	
P6021400PCT/IN	AMT-P110	India	Amsterdam Molecular Therapeutics (AMT) B.V.;Proyecto de Biomedicina CIMA S.L.	Porphobilinogen deaminase gene therapy	US-2012-0178646-A1	Abandoned	29-Sep-2009	
P6021400PCT/JP	AMT-P110	Japan	Amsterdam Molecular Therapeutics (AMT) B.V.;Proyecto de Biomedicina CIMA S.L.	Porphobilinogen deaminase gene therapy	2011-528967	Abandoned	29-Sep-2009	
P6021400PCT/KR	AMT-P110	Republic of Korea	Amsterdam Molecular Therapeutics (AMT) B.V.;Proyecto de Biomedicina CIMA S.L.	Porphobilinogen deaminase gene therapy	2011-7009883	Pending	29-Sep-2009	
P6021400PCT/MX	AMT-P110	Mexico	Amsterdam Molecular Therapeutics (AMT) B.V.;Proyecto de Biomedicina CIMA S.L.	Porphobilinogen deaminase gene therapy	MX/a/2011/003229	Published	29-Sep-2009	
P6021400PCT/US	AMT-P110	United States of America	Amsterdam Molecular Therapeutics (AMT)	Porphobilinogen deaminase gene therapy	US-2011-0262399	Pending	29-Sep-2009	

Case Ref.	UniQure Ref.	Country	Owners	Title	Official No.	Case Status	Filing Date	Date Registration
			B.V.;Proyecto de Biomedicina CIMA S.L.					
P6021400PCT/ZA	AMT-P110	South Africa	Amsterdam Molecular Therapeutics (AMT) B.V.;Proyecto de Biomedicina CIMA S.L.	Porphobilinogen deaminase gene therapy	2011/02287	Pending	29-Sep-2009	
P6021400US	AMT-P110	United States of America	Amsterdam Molecular Therapeutics B.V.;Proyecto de Biomedicina CIMA S.L.	Porphobilinogen deaminase gene therapy	61/100,881	abandoned	29-Sep-2008	
P6022296EP	AMT-P111	Europe	Amsterdam Molecular Therapeutics (AMT) IP B.V.	Alanine-glyoxylate aminotransferase therapeutics	09151795.3	Withdraw Before Publication	30-Jan-2009	
P6022296PCT	AMT-P111	PCT	Amsterdam Molecular Therapeutics (AMT) IP B.V.	Alanine-glyoxylate aminotransferase therapeutics	PCT/NL2010/050044	converted	01-Feb-2010	
P6022296PCT/CA	AMT-P111	Canada	Amsterdam Molecular Therapeutics (AMT) IP B.V.	Alanine-glyoxylate aminotransferase therapeutics	2,750,811	Pending	01-Feb-2010	
P6022296PCT/EP	AMT-P111	Europe	uniQure IP B.V.	Alanine-glyoxylate aminotransferase therapeutics	2384200	Notice of 71(3) Received	01-Feb-2010	
P6022296PCT/JP	AMT-P111	Japan	Amsterdam Molecular Therapeutics (AMT) IP B.V.	Alanine-glyoxylate aminotransferase therapeutics	2011-547843	Examination Requested	01-Feb-2010	
P6022296PCT/US	AMT-P111	United States of	Amsterdam Molecular	Alanine-glyoxylate	13/146,869	Pending	01-Feb-2010	

P6030610EP	AMT-P119EPP0	America	Therapeutics (AMT) IP B.V.	aminotransferase therapeutics			
P6030610PCT	AMT-P119WO	Europe	Amsterdam Molecular Therapeutics (AMT) IP B.V.	Monomeric duplex AAV vectors	10159030.5	Withdraw Before Publication	01-Apr-2010
P6030610US	AMT-P119USP0	PCT	uniQure IP B.V.	Monomeric duplex AAV vectors	PCT/NL2011/050221	Abandoned	01-Apr-2011
P6031130PCT	AMT-P112WO	United States of America	Amsterdam Molecular Therapeutics (AMT) IP B.V.	Monomeric duplex AAV vectors	61/319,984	Abandoned	01-Apr-2010
P6031130PCT/EP	AMT-P112WO	PCT	Amsterdam Molecular Therapeutics (AMT) IP B.V.;de Wal, Janneke;Gaudet, Daniel	Use of lipoprotein lipase (LPL) in therapy	PCT/NL2010/050294	Abandoned	18-May-2010
P6031130PCT/US	AMT-P112WO	Europe	Amsterdam Molecular Therapeutics (AMT) IP B.V.	Use of lipoprotein lipase (LPL) in therapy	10732461.8	Abandoned	18-May-2010
P6031346EP	AMT-P120EPP0	United States of America	Amsterdam Molecular Therapeutics (AMT) IP B.V.	Use of lipoprotein lipase (LPL) in therapy	13/321,491	Abandoned	18-May-2010
P6031346PCT	AMT-P120EPP0	Europe	Amsterdam Molecular Therapeutics (AMT) IP B.V.	Method for determining efficacy of therapy and	10164745.1	Abandoned	02-Jun-2010

Case Ref.	UniQure Ref.	Country	Owners	Title	Official No.	Case Status	Filing Date	Date Registration
P6031346PCT	AMT-P120EPP0	PCT	B.V. uniQure IP B.V.	determining presence or risk of disease Method for determining efficacy of therapy and determining presence or risk of disease	PCT/NL2011/050399	Abandoned	06-Jun-2011	
P6031346US	AMT-P120USP0	United States of America	Amsterdam Molecular Therapeutics (AMT) IP B.V.	Method for determining efficacy of therapy and determining presence or risk of disease	12/792,067	Abandoned	02-Jun-2010	
P6034703PCT	AMT-P117WO	PCT	uniQure IP B.V.	Mutated Rep encoding sequences for use in AAV production	PCT/NL2011/050170	Abandoned	11-Mar-2011	
P6034703PCT/EP	AMT-P117	Europe	uniQure IP B.V.	Mutated Rep encoding sequences for use in AAV production	2545165	Published	11-Mar-2011	
P6034703PCT/US	AMT-P117	United States of America	uniQure IP B.V.	Mutated Rep encoding sequences for use in AAV production	13/583,920	Published	11-Mar-2011	
P6034704PCT	AMT-P118WO	PCT	uniQure IP B.V.	Method for identifying variant Rep protein encoding nucleic acids	PCT/NL2011/050171	abandoned	11-Mar-2011	
P6035462EP	AMT-P121	Europe	Amsterdam Molecular Therapeutics (AMT) IP B.V.	Method for the preparation of cells	11160727.1	Withdraw Before Publication	31-Mar-2011	
P6035462US	AMT-P121	United States of America	Amsterdam Molecular Therapeutics (AMT) IP B.V.	Method for the preparation of cells	61/470,033	Abandoned	31-Mar-2011	
P6036755EP	AMT-P113EPP0	Europe	uniQure IP B.V.	Removal of contaminating viruses from AAV preparations	11180594.1	Abandoned	08-Sep-2011	
P6036755PCT	AMT-P113EPP0	PCT	uniQure IP B.V.	Removal of contaminating viruses from AAV preparations	PCT/NL2012/050619	Pending	07-Sep-2012	
P6036755US	AMT-P113USP0	United States of America	uniQure IP B.V.	Removal of contaminating viruses from AAV preparations	61/532,176	abandoned	08-Sep-2011	
P6042305EP	AMT-P122	Europe	uniQure IP B.V.	DNA impurities		Not Yet Filed		
P6042305US	AMT-P122	United States of America	uniQure IP B.V.	DNA impurities		Not Yet Filed		

Elzas Noordzij B.V.







Catchword	Type	Country	Classes	Appl.No.	Appl.date	Reg.No.	Reg.date	Ren.date	Applicant	Status	Case No.	Watch
AMT	Wordmark	BX	01, 05, 42	996846	13-9-01.	696184	13-9-01.	13-9-11.	AMT B.V.	Registered	T17138BX00	NO
AMT	Wordmark	CA	01, 05, 42	1130879	12-2-02.	630501	19-1-05.	19-1-20.	AMT B.V.	Registered	T17138CA00	NO
AMT	Wordmark	EU	01, 05, 42	2573137	11-2-02.	2573137	3-7-03.	11-2-12.	Amsterdam Molecular Therapeutics (AMT) Holding N.V.	Registered	T17138EU00	NO
	Logotype	BX	01, 05, 42	996845	13-9-01.	700080	13-9-01.	13-9-11.	AMT B.V.	Registered	T17139BX00	NO
AMT	Wordmark	CA	01, 05, 42, 44	1478301	23-4-10.				Amsterdam Molecular Therapeutics (AMT) IP B.V.	Pending	T55829CA00	Yes
AMT	Wordmark	EU	01, 05, 42, 44	8640237	26-10-09.	8640237	10-5-10.	26-10-19.	Amsterdam Molecular Therapeutics (AMT) IP B.V.	Registered	T55829EU00	Yes
AMT	Wordmark	US	01, 05, 42, 44	85/021857	23-4-10.				Amsterdam Molecular Therapeutics (AMT) IP B.V.	Pending	T55829US00	Yes
AMT	Wordmark	CH (WO)	01, 05, 42, 44	8640237-01	23-4-10.	1040425	23-4-10.	23-4-20.	Amsterdam Molecular Therapeutics (AMT) IP B.V.	Registered	T55829WO00	Yes
	Logotype	CH	05	536152008	6-4-09.	587323	9-6-09.	6-4-19.	Amsterdam Molecular Therapeutics (AMT) Holding N.V.	Registered	T56005CH00	No
	Logotype	IL	05	209906	24-3-08.	209906	7-2-10.	24-3-18.	Amsterdam Molecular	Registered	T56005IL00	No



Therapeutics (AMT)  
Holding N.V.


Logotype	IS	05	10692009	22-4-09.	3772009	2-6-09.	2-6-19.	Amsterdam Molecular Therapeutics (AMT) Holding N.V.	Registered	T56005IS00	No
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Catchword	Type	Country	Classes	Appl.No.	Appl.date	Reg.No.	Reg.date	Ren.date	Applicant	Status	Case No.	Watch
	Logotype	JO	05	100494	23-4-08.	100494	23-4-08.	23-4-18.	Amsterdam Molecular Therapeutics (AMT) Holding N.V.	Registered	T56005JO00	No
	Logotype	NO	05	200905089	21-4-09.	251774	14-7-09.	14-7-19.	Amsterdam Molecular Therapeutics (AMT) Holding N.V.	Registered	T56005NO00	No
	Logotype	TR	05	200925333	18-5-09.	200925333	4-5-10.	18-5-19.	Amsterdam Molecular Therapeutics (AMT) Holding N.V.	Registered	T56005TR00	No
	Logotype	EU	01, 05, 42, 44	8640252	26-10-09.	8640252	10-5-10.	26-10-19.	Amsterdam Molecular Therapeutics (AMT) IP B.V.	Registered	T55830EU00	Yes
	Logotype	US	01, 05, 42, 44	85/021908	23-4-10.				Amsterdam Molecular Therapeutics (AMT) IP B.V.	Pending	T55830US00	Yes
	Wordmark	EU	01, 05, 42, 44	9599937	15-12-10.				Amsterdam Molecular Therapeutics (AMT) IP B.V.	Pending	T56839EU00	
DELIVERING CURE	Wordmark	AE	05	113972	3-6-08.				Amsterdam Molecular Therapeutics (AMT) Holding N.V.	Pending	T56004AE00	No
DELIVERING CURE	Wordmark	BH	05	64727	18-3-08.				Amsterdam Molecular Therapeutics (AMT) Holding N.V.	Pending	T56004BH00	No

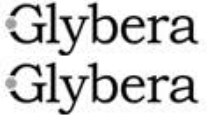
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Catchword	Type	Country	Classes	Appl.No.	Appl.date	Reg.No.	Reg.date	Ren.date	Applicant	Status	Case No.	Watch
DELIVERING CURE	Wordmark	CA	5	1388257	20-3-08.				Amsterdam Molecular Therapeutics (AMT) Holding N.V.	Pending	T56004CA00	No
DELIVERING CURE	Wordmark	IR	05	86122678	18-3-08.	157479	14-9-08.	18-3-18.	Amsterdam Molecular Therapeutics (AMT) Holding N.V.	Registered	T56004IR00	No
DELIVERING CURE	Wordmark	JP	05	2008023029	27-3-08.	5343913	6-8-10.	6-8-20.	Amsterdam Molecular Therapeutics (AMT) Holding N.V.	Registered	T56004JP00	No
DELIVERING CURE	Wordmark	LB	05	2449	8-4-08.	116062	24-4-08.	24-4-23.	Amsterdam Molecular Therapeutics (AMT) Holding N.V.	Registered	T56004LB00	No
DELIVERING CURE	Wordmark	LY	05	17093	5-2-09.				Amsterdam Molecular Therapeutics (AMT) Holding N.V.	Pending	T56004LY00	No
DELIVERING CURE	Wordmark	MA	05	118083	19-6-08.	118083	17-11-08.	19-6-18.	Amsterdam Molecular Therapeutics (AMT) Holding N.V.	Registered	T56004MA00	No
DELIVERING CURE	Wordmark	OM	05	49398	19-3-08.	49398	11-8-09.	19-3-18.	Amsterdam Molecular Therapeutics (AMT) Holding N.V.	Registered	T56004OM00	No
DELIVERING CURE	Wordmark	QA	05	50165	3-4-08.				Amsterdam Molecular Therapeutics (AMT) Holding N.V.	Pending	T56004QA00	No
DELIVERING CURE	Wordmark	RU	05	2008707490	14-3-08.	381651	16-6-09.	14-3-18.	Amsterdam Molecular Therapeutics (AMT) Holding N.V.	Registered	T56004RU00	No
DELIVERING CURE	Wordmark	SY	05	3814	22-4-08.				Amsterdam Molecular Therapeutics (AMT) Holding N.V.	Pending	T56004SY00	No
DELIVERING CURE	Wordmark	TN	05	EE080755	19-3-08.	EE080755	26-1-10.	19-3-18.	Amsterdam Molecular Therapeutics (AMT) Holding N.V.	Registered	T56004TN00	No
DELIVERING CURE	Wordmark	US	05	77/421590	13-3-08.				Amsterdam Molecular Therapeutics (AMT) Holding N.V.	Pending	T56004US00	No
DELIVERING CURE	Wordmark	ZA	05	200805836	14-3-08.	200805836	14-3-08.	14-3-18.	Amsterdam Molecular Therapeutics (AMT)	Registered	T56004ZA00	No

Catchword	Type	Country	Classes	Appl.No.	Appl.date	Reg.No.	Reg.date	Ren.date	Applicant	Status	Case No.	Watch
	Logotype	EU	05, 44	8640609	26-10-09.	8640609	10-5-10.	26-10-19.	Amsterdam Molecular Therapeutics (AMT) IP B.V.	Registered	T55831EU00	Yes
	Logotype	US	05, 44	85/021938	23-4-10.				Amsterdam Molecular Therapeutics (AMT) IP B.V.	Pending	T55831US00	Yes
GLYBERA	Wordmark	AE	05	101941	31-10-07.				Amsterdam Molecular Therapeutics (AMT) Holding N.V.	Pending	T56001AE00	Yes
GLYBERA	Wordmark	AU	05	1176048	14-5-07.	1176048	12-12-07.	14-5-17.	Amsterdam Molecular Therapeutics (AMT) Holding N.V.	Registered	T56001AU00	Yes
GLYBERA	Wordmark	BH	05	62689	7-1-08.				Amsterdam Molecular Therapeutics (AMT) Holding N.V.	Pending	T56001BH00	Yes
GLYBERA	Wordmark	CA	5	1355754	16-7-07.				Amsterdam Molecular Therapeutics (AMT) Holding N.V.	Pending	T56001CA00	Yes
GLYBERA	Wordmark	CH	05	551392007	14-5-07.	562178	11-9-07.	14-5-17.	Amsterdam Molecular Therapeutics (AMT) Holding N.V.	Registered	T56001CH00	Yes
GLYBERA	Wordmark	DZ	05	72791	24-10-07.				Amsterdam Molecular Therapeutics (AMT) Holding N.V.	Pending	T56001DZ00	Yes
GLYBERA	Wordmark	EG	05	208229	22-10-07.				Amsterdam Molecular Therapeutics (AMT) Holding N.V.	Pending	T56001EG00	Yes
GLYBERA	Wordmark	EU	05, 44	5901269	1-5-07.	5901269	14-5-09.	1-5-17.	Amsterdam Molecular Therapeutics (AMT) Holding N.V.	Registered	T56001EU00	Yes
GLYBERA	Wordmark	IL	05	204800	21-10-07.	204800	11-8-09.	21-10-17.	Amsterdam Molecular Therapeutics (AMT) Holding N.V.	Registered	T56001IL00	Yes
GLYBERA	Wordmark	IS	05	14642007	14-5-07.	8122007	4-7-07.	4-7-17.	Amsterdam Molecular Therapeutics (AMT) Holding N.V.	Registered	T56001IS00	Yes

Catchword	Type	Country	Classes	Appl.No.	Appl.date	Reg.No.	Reg.date	Ren.date	Applicant	Status	Case No.	Watch
GLYBERA	Wordmark	JO	05	99133	24-10-07.	99133	1-5-07.	1-5-17.	Amsterdam Molecular Therapeutics (AMT) Holding N.V.	Registered	T56001JO00	Yes
GLYBERA	Wordmark	JP	05	2007054257	30-5-07.	5088657	2-11-07.	2-11-17.	Amsterdam Molecular Therapeutics (AMT) Holding N.V.	Registered	T56001JP00	Yes
GLYBERA	Wordmark	LB	05	6612	23-10-07.	113370	25-10-07.	25-10-22.	Amsterdam Molecular Therapeutics (AMT) Holding N.V.	Registered	T56001LB00	Yes
GLYBERA	Wordmark	LY	05	16593	22-12-08.				Amsterdam Molecular Therapeutics (AMT) Holding N.V.	Pending	T56001LY00	Yes
GLYBERA	Wordmark	MA	05	113550	23-10-07.	113550	23-10-07.	23-10-17.	Amsterdam Molecular Therapeutics (AMT) Holding N.V.	Registered	T56001MA00	Yes
GLYBERA	Wordmark	NO	05	200705606	15-5-07.	241553	19-10-07.	19-10-17.	Amsterdam Molecular Therapeutics (AMT) Holding N.V.	Registered	T56001NO00	Yes
GLYBERA	Wordmark	NZ	05	768310	14-5-07.	768310	15-11-07.	14-5-17.	Amsterdam Molecular Therapeutics (AMT) Holding N.V.	Registered	T56001NZ00	Yes
GLYBERA	Wordmark	OM	05	47462	22-10-07.	47462	24-8-08.	22-10-17.	Amsterdam Molecular Therapeutics (AMT) Holding N.V.	Registered	T56001OM00	Yes
GLYBERA	Wordmark	QA	05	47253	31-10-07.	47253	31-12-09.	31-10-17.	Amsterdam Molecular Therapeutics (AMT) Holding N.V.	Registered	T56001QA00	Yes
GLYBERA	Wordmark	RU	05	2008707340	13-3-08.	377215	20-4-09.	13-3-18.	Amsterdam Molecular Therapeutics (AMT) Holding N.V.	Registered	T56001RU00	Yes
GLYBERA	Wordmark	SA	05	125692	12-1-08.				Amsterdam Molecular Therapeutics (AMT) Holding N.V.	Pending	T56001SA00	Yes
GLYBERA	Wordmark	SY	05	4268	28-10-07.				Amsterdam Molecular Therapeutics (AMT) Holding N.V.	Pending	T56001SY00	Yes
GLYBERA	Wordmark	TN	05	EE072667	24-10-07.	EE072667	19-5-09.	24-10-17.	Amsterdam Molecular Therapeutics (AMT) Holding N.V.	Registered	T56001TN00	Yes

Catchword	Type	Country	Classes	Appl.No.	Appl.date	Reg.No.	Reg.date	Ren.date	Applicant	Status	Case No.	Watch
GLYBERA	Wordmark	TR	05	2007026778	17-5-07.	200726778	17-5-07.	17-5-17.	Amsterdam Molecular Therapeutics (AMT) Holding N.V.	Registered	T56001TR00	Yes
GLYBERA	Wordmark	US	05	77/179356	11-5-07.				Amsterdam Molecular Therapeutics (AMT) Holding N.V.	Pending	T56001US00	Yes
GLYBERA	Wordmark	ZA	05	200723919	19-10-07.	2007/23919	19-10-07.	19-10-17.	Amsterdam Molecular Therapeutics (AMT) Holding N.V.	Registered	T56001ZA00	Yes
	Logotype	EU	05, 44	8640641	26-10-09.	8640641	10-5-10.	26-10-19.	Amsterdam Molecular	Registered	T55832EU00	Indirect watch

										Therapeutics (AMT) IP B.V.			
	Logotype	US	05, 44	85/021985	23-4-10.					Amsterdam Molecular Therapeutics (AMT) IP B.V.	Pending	T55832US00	Indirect watch
LPLCHIP	Wordmark	CA	1, 10, 42, 44, 5, 9	1474070	22-3-10.					Amsterdam Molecular Therapeutics (AMT) IP B.V.	Pending	T55770CA00	Yes
LPLCHIP	Wordmark	EU	01, 05, 09, 10, 42, 44	8590911	2-10-09.	8590911	31-5-10.	2-10-19.		Amsterdam Molecular Therapeutics (AMT) IP B.V.	Registered	T55770EU00	Yes
LPLCHIP	Wordmark	US	1, 5, 9	77/964892	22-3-10.					Amsterdam Molecular Therapeutics (AMT) IP B.V.	Pending	T55770US00	Yes
LPLCHIP	Wordmark	CH (WO)	01, 05, 09, 10	8590911-01	6-4-10.	1036745	6-4-10.	6-4-20.		Amsterdam Molecular Therapeutics (AMT) IP B.V.	Registered	T55770WO00	Yes
LPLCHIP	Wordmark	IS (WO)	01, 05, 09, 10	8590911-01	6-4-10.	1036745	6-4-10.	6-4-20.		Amsterdam Molecular Therapeutics (AMT) IP B.V.	Registered	T55770WO00	Yes
LPLCHIP	Wordmark	NO (WO)	01, 05, 09, 10	8590911-01	6-4-10.	1036745	6-4-10.	6-4-20.		Amsterdam Molecular Therapeutics (AMT) IP B.V.	Registered	T55770WO00	Yes
VECTIPRO	Wordmark	AE	05	101942	31-10-07.					Amsterdam Molecular Therapeutics (AMT) Holding N.V.	Pending	T56002AE00	Yes
VECTIPRO	Wordmark	AU	05	1176051	14-5-07.	1176051	12-12-07.	14-5-17.		Amsterdam Molecular Therapeutics (AMT) Holding N.V.	Registered	T56002AU00	Yes

Catchword	Type	Country	Classes	Appl.No.	Appl.date	Reg.No.	Reg.date	Ren.date	Applicant	Status	Case No.	Watch
VECTIPRO	Wordmark	BH	05	62690	7-1-08.				Amsterdam Molecular Therapeutics (AMT) Holding N.V.	Pending	T56002BH00	Yes
VECTIPRO	Wordmark	CA	5	1355761	16-7-07.				Amsterdam Molecular Therapeutics (AMT) Holding N.V.	Pending	T56002CA00	Yes
VECTIPRO	Wordmark	CH	05	551382007	14-5-07.	562177	11-9-07.	14-5-17.	Amsterdam Molecular Therapeutics (AMT) Holding N.V.	Registered	T56002CH00	Yes
VECTIPRO	Wordmark	DZ	05	72793	24-10-07.				Amsterdam Molecular Therapeutics (AMT) Holding N.V.	Pending	T56002DZ00	Yes
VECTIPRO	Wordmark	EG	05	208203	22-10-07.				Amsterdam Molecular Therapeutics (AMT) Holding N.V.	Pending	T56002EG00	Yes
VECTIPRO	Wordmark	EU	05	5901277	1-5-07.	5901277	10-4-08.	1-5-17.	Amsterdam Molecular Therapeutics (AMT) Holding N.V.	Registered	T56002EU00	Yes
VECTIPRO	Wordmark	IL	05	204915	23-10-07.	204915	11-8-09.	23-10-17.	Amsterdam Molecular Therapeutics (AMT) Holding N.V.	Registered	T56002IL00	Yes
VECTIPRO	Wordmark	IR	05	86091403	8-12-07.	157475	14-9-08.	8-12-17.	Amsterdam Molecular Therapeutics (AMT) Holding N.V.	Registered	T56002IR00	Yes
VECTIPRO	Wordmark	IS	05	14632007	14-5-07.	8112007	4-7-07.	4-7-17.	Amsterdam Molecular Therapeutics (AMT) Holding N.V.	Registered	T56002IS00	Yes
VECTIPRO	Wordmark	JO	05	99366	24-10-07.	99366	14-1-09.	1-5-17.	Amsterdam Molecular Therapeutics (AMT) Holding N.V.	Registered	T56002JO00	Yes
VECTIPRO	Wordmark	JP	05	2007054258	30-5-07.	5088658	2-11-07.	2-11-17.	Amsterdam Molecular Therapeutics (AMT) Holding N.V.	Registered	T56002JP00	Yes
VECTIPRO	Wordmark	LB	05	6622	23-10-07.	113434	30-10-07.	30-10-22.	Amsterdam Molecular Therapeutics (AMT) Holding N.V.	Registered	T56002LB00	Yes
VECTIPRO	Wordmark	LY	05	16595	22-12-08.				Amsterdam Molecular Therapeutics (AMT) Holding N.V.	Pending	T56002LY00	Yes

Catchword	Type	Country	Classes	Appl.No.	Appl.date	Reg.No.	Reg.date	Ren.date	Applicant	Status	Case No.	Watch
VECTIPRO	Wordmark	MA	05	113551	23-10-07.	113551	23-10-07.	23-10-17.	Amsterdam Molecular Therapeutics (AMT) Holding N.V.	Registered	T56002MA00	Yes
VECTIPRO	Wordmark	NO	05	200705604	15-5-07.	241558	22-10-07.	22-10-17.	Amsterdam Molecular Therapeutics (AMT) Holding N.V.	Registered	T56002NO00	Yes
VECTIPRO	Wordmark	NZ	05	768309	14-5-07.	768309	12-2-09.	1-5-17.	Amsterdam Molecular Therapeutics (AMT) Holding N.V.	Registered	T56002NZ00	Yes
VECTIPRO	Wordmark	OM	05	47461	22-10-07.	47461	30-5-09.	22-10-17.	Amsterdam Molecular Therapeutics (AMT) Holding N.V.	Registered	T56002OM00	Yes
VECTIPRO	Wordmark	QA	05	47255	31-10-07.	47255	31-12-09.	31-10-17.	Amsterdam Molecular Therapeutics (AMT) Holding N.V.	Pending	T56002QA00	Yes
VECTIPRO	Wordmark	RU	05	2008707342	13-3-08.	381400	10-6-09.	13-3-18.	Amsterdam Molecular Therapeutics (AMT) Holding N.V.	Registered	T56002RU00	Yes
VECTIPRO	Wordmark	SA	05	125693	12-1-08.				Amsterdam Molecular Therapeutics (AMT) Holding N.V.	Pending	T56002SA00	Yes
VECTIPRO	Wordmark	SY	05	4269	28-10-07.				Amsterdam Molecular	Pending	T56002SY00	Yes



VECTIPRO	Wordmark	TN	05	EE072666	24-10-07.	EE072666	19-5-09.	24-10-17.	Therapeutics (AMT) Holding N.V. Amsterdam Molecular Therapeutics (AMT) Holding N.V.	Registered	T56002TN00	Yes
VECTIPRO	Wordmark	TR	05	2007026779	17-5-07.	200726779	17-5-07.	17-5-17.	Amsterdam Molecular Therapeutics (AMT) Holding N.V.	Registered	T56002TR00	Yes
VECTIPRO	Wordmark	US	05	77/179357	11-5-07.	3703954	3-11-09.	3-11-19.	Amsterdam Molecular Therapeutics (AMT) Holding N.V.	Registered	T56002US00	Yes
VECTIPRO	Wordmark	ZA	05	200723918	19-10-07.	2007/23918	19-10-07.	19-10-17.	Amsterdam Molecular Therapeutics (AMT) Holding N.V.	Registered	T56002ZA00	Yes
ZYAMTIN	Wordmark	AE	05	101943	31-10-07.				Amsterdam Molecular Therapeutics (AMT) Holding N.V.	Pending	T56003AE00	Yes

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Catchword	Type	Country	Classes	Appl.No.	Appl.date	Reg.No.	Reg.date	Ren.date	Applicant	Status	Case No.	Watch
ZYAMTIN	Wordmark	AU	05	1176049	14-5-07.	1176049	12-12-07.	14-5-17.	Amsterdam Molecular Therapeutics (AMT) Holding N.V.	Registered	T56003AU00	Yes
ZYAMTIN	Wordmark	BH	05	62691	7-1-08.				Amsterdam Molecular Therapeutics (AMT) Holding N.V.	Pending	T56003BH00	Yes
ZYAMTIN	Wordmark	CA	5	1355762	16-7-07.				Amsterdam Molecular Therapeutics (AMT) Holding N.V.	Pending	T56003CA00	Yes
ZYAMTIN	Wordmark	CH	05	551982007	15-5-07.	562360	13-9-07.	15-5-17.	Amsterdam Molecular Therapeutics (AMT) Holding N.V.	Registered	T56003CH00	Yes
ZYAMTIN	Wordmark	DZ	05	72792	24-10-07.				Amsterdam Molecular Therapeutics (AMT) Holding N.V.	Pending	T56003DZ00	Yes
ZYAMTIN	Wordmark	EG	05	208231	22-10-07.				Amsterdam Molecular Therapeutics (AMT) Holding N.V.	Pending	T56003EG00	Yes
ZYAMTIN	Wordmark	EU	05, 44	5901251	1-5-07.	5901251	22-1-09.	1-5-17.	Amsterdam Molecular Therapeutics (AMT) Holding N.V.	Registered	T56003EU00	Yes
ZYAMTIN	Wordmark	IL	05	204799	21-10-07.	204799	11-4-09.	21-10-17.	Amsterdam Molecular Therapeutics (AMT) Holding N.V.	Registered	T56003IL00	Yes
ZYAMTIN	Wordmark	IR	05	86091401	8-12-07.	158201	14-9-08.	8-12-17.	Amsterdam Molecular Therapeutics (AMT) Holding N.V.	Registered	T56003IR00	Yes
ZYAMTIN	Wordmark	IS	05	14652007	14-5-07.	8132007	4-7-07.	4-7-17.	Amsterdam Molecular Therapeutics (AMT) Holding N.V.	Registered	T56003IS00	Yes
ZYAMTIN	Wordmark	JO	05	99208	24-10-07.	99208	3-3-09.	1-5-17.	Amsterdam Molecular Therapeutics (AMT) Holding N.V.	Registered	T56003JO00	Yes
ZYAMTIN	Wordmark	JP	05	2007054259	30-5-07.	5088659	2-11-07.	2-11-17.	Amsterdam Molecular Therapeutics (AMT) Holding N.V.	Registered	T56003JP00	Yes
ZYAMTIN	Wordmark	LB	05	6623	23-10-07.	113437	30-10-07.	30-10-22.	Amsterdam Molecular Therapeutics (AMT) Holding N.V.	Registered	T56003LB00	Yes

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Catchword	Type	Country	Classes	Appl.No.	Appl.date	Reg.No.	Reg.date	Ren.date	Applicant	Status	Case No.	Watch
ZYAMTIN	Wordmark	LY	05	16594	22-12-08.				Amsterdam Molecular Therapeutics (AMT) Holding N.V.	Pending	T56003LY00	Yes
ZYAMTIN	Wordmark	MA	05	113552	23-10-07.	113552	23-10-07.	23-10-17.	Amsterdam Molecular Therapeutics (AMT) Holding N.V.	Registered	T56003MA00	Yes
ZYAMTIN	Wordmark	NO	05	200705605	15-5-07.	241517	18-10-07.	18-10-17.	Amsterdam Molecular Therapeutics (AMT) Holding N.V.	Registered	T56003NO00	Yes
ZYAMTIN	Wordmark	NZ	05	768311	14-5-07.	768311	15-11-07.	14-5-17.	Amsterdam Molecular Therapeutics (AMT) Holding N.V.	Registered	T56003NZ00	Yes
ZYAMTIN	Wordmark	OM	05	47463	22-10-07.	47463	30-5-09.	22-10-17.	Amsterdam Molecular Therapeutics (AMT) Holding N.V.	Registered	T56003OM00	Yes
ZYAMTIN	Wordmark	QA	05	47254	31-10-07.	47254	31-12-09.	31-10-17.	Amsterdam Molecular Therapeutics (AMT) Holding N.V.	Registered	T56003QA00	Yes
ZYAMTIN	Wordmark	RU	05	2008707341	13-3-08.	394999	1-12-09.	13-3-18.	Amsterdam Molecular Therapeutics (AMT) Holding N.V.	Registered	T56003RU00	Yes
ZYAMTIN	Wordmark	SA	05	125694	12-1-08.				Amsterdam Molecular Therapeutics (AMT) Holding N.V.	Pending	T56003SA00	Yes
ZYAMTIN	Wordmark	SY	05	4267	28-10-07.				Amsterdam Molecular Therapeutics (AMT) Holding N.V.	Pending	T56003SY00	Yes
ZYAMTIN	Wordmark	TN	05	EE072668	24-10-07.	EE072668	19-5-09.	24-10-17.	Amsterdam Molecular Therapeutics (AMT) Holding N.V.	Registered	T56003TN00	Yes
ZYAMTIN	Wordmark	TR	05	2007026780	17-5-07.	200726780	7-4-08.	17-5-17.	Amsterdam Molecular Therapeutics (AMT) Holding N.V.	Registered	T56003TR00	Yes
ZYAMTIN	Wordmark	US	05	77/179359	11-5-07.	3855311	5-10-10.	5-10-20.	Amsterdam Molecular Therapeutics (AMT) Holding N.V.	Registered	T56003US00	Yes
ZYAMTIN	Wordmark	ZA	05	200723917	19-10-07.	200723917	14-7-10.	19-10-17.	Amsterdam Molecular Therapeutics (AMT) Holding N.V.	Registered	T56003ZA00	Yes

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EXHIBIT E

BORROWER'S DEPOSIT ACCOUNTS AND INVESTMENT ACCOUNTS

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EXHIBIT F

COMPLIANCE CERTIFICATE

Hercules Technology Growth Capital, Inc.  
400 Hamilton Avenue, Suite 310  
Palo Alto, CA 94301

Reference is made to that certain Loan and Security Agreement dated [ ], 2013 and all ancillary documents entered into in connection with such Loan and Security Agreement all as may be amended from time to time, (hereinafter referred to collectively as the "Loan Agreement") between Hercules Technology Growth Capital, Inc. as Lender and (i) UNIQUE BIOPHARMA B.V., a ("uniQure"), (ii) UNIQUE IP B.V., a ("uniQure IP"), (iii) each of the subsidiaries of uniQure (uniQure, uniQure IP and such subsidiaries are hereinafter collectively referred to as "Borrower"), as Borrower. All capitalized terms not defined herein shall have the same meaning as defined in the Loan Agreement.

The undersigned is an Officer of the Borrower, knowledgeable of all Borrower's financial matters, and is authorized to provide certification of information regarding the Borrower; hereby certifies that in accordance with the terms and conditions of the Loan Agreement, the Borrower is in compliance for the period ending of all covenants, conditions and terms and hereby reaffirms that all representations and warranties contained therein are true and correct in all material respects on and as of the date of this Compliance Certificate with the same effect as though made on and as of such date, except to the extent such representations and warranties expressly relate to an earlier date, after giving effect in all cases to any standard(s) of materiality contained in the Loan Agreement as to such representations and warranties. Attached are the required documents supporting the above certification. The undersigned further certifies that these are prepared in accordance with IFRS (except for the absence of footnotes with respect to unaudited financial statement and subject to normal year-end adjustments) and are consistent from one period to the next except as explained below.

REPORTING REQUIREMENT	REQUIRED	CHECK IF ATTACHED
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Interim Financial Statements	Monthly within 30 days	
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Interim Financial Statements	Quarterly within 45 days	
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Audited Financial Statements	FYE within 150 days	
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Very Truly Yours,

UNIQUE BIOPHARMA B.V.

By: \_\_\_\_\_

Name: \_\_\_\_\_

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Its: \_\_\_\_\_

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EXHIBIT G

FORM OF JOINDER AGREEMENT

This Joinder Agreement (the "Joinder Agreement") is made and dated as of [ ], 20[ ], and is entered into by and between , a corporation ("Subsidiary"), and HERCULES TECHNOLOGY GROWTH CAPITAL, INC., a Maryland corporation, as a Lender.

RECITALS

A. Subsidiary's Affiliates, (i) UNIQUE BIOPHARMA B.V., a ("uniQure"), (ii) UNIQUE IP B.V., a ("uniQure IP"), (iii) each of the subsidiaries of uniQure (uniQure, uniQure IP and such subsidiaries are hereinafter collectively referred to as "Borrower") [have entered/desires to enter] into that certain Loan and Security Agreement dated [ ], 2013, with Lender, as such agreement may be amended (the "Loan Agreement"), together with the other agreements executed and delivered in connection therewith;

B. Subsidiary acknowledges and agrees that it will benefit both directly and indirectly from Borrower's execution of the Loan Agreement and the other agreements executed and delivered in connection therewith;

AGREEMENT



NOW THEREFORE, Subsidiary and Lender agree as follows:

1. The recitals set forth above are incorporated into and made part of this Joinder Agreement. Capitalized terms not defined herein shall have the meaning provided in the Loan Agreement.
2. By signing this Joinder Agreement, Subsidiary shall be bound by the terms and conditions of the Loan Agreement the same as if it were the Borrower (as defined in the Loan Agreement) under the Loan Agreement, mutatis mutandis, provided however, that Lender shall have no duties, responsibilities or obligations to Subsidiary arising under or related to the Loan Agreement or the other agreements executed and delivered in connection therewith. Rather, to the extent that Lender has any duties, responsibilities or obligations arising under or related to the Loan Agreement or the other agreements executed and delivered in connection therewith, those duties, responsibilities or obligations shall flow only to Borrower and not to Subsidiary or any other person or entity. By way of example (and not an exclusive list): (a) Lender’s providing notice to Borrower in accordance with the Loan Agreement or as otherwise agreed between Borrower and Lender shall be deemed provided to Subsidiary; (b) a Lender’s providing an Advance to Borrower shall be deemed an Advance to Subsidiary; and (c) Subsidiary shall have no right to request an Advance or make any other demand on Lender.

[REMAINDER OF PAGE INTENTIONALLY LEFT BLANK]

[SIGNATURE PAGE TO JOINDER AGREEMENT]

SUBSIDIARY:

By:  
Name:  
Title:  
Address:

Telephone:  
Facsimile:

HERCULES TECHNOLOGY GROWTH CAPITAL, INC.

By: \_\_\_\_\_  
Name: \_\_\_\_\_  
Title: \_\_\_\_\_

Address:  
400 Hamilton Ave., Suite 310  
Palo Alto, CA 94301  
Facsimile: 650-473-9194  
Telephone: 650-289-3060

EXHIBIT H

ACH DEBIT AUTHORIZATION AGREEMENT

Hercules Technology Growth Capital, Inc.  
400 Hamilton Avenue, Suite 310  
Palo Alto, CA 94301

Re: Loan and Security Agreement dated \_\_\_\_\_, 2013 between (i) UNIQUE BIOPHARMA B.V., a (“uniQure”),  
(ii) UNIQUE IP B.V., a (“uniQure IP”), (iii) each of the subsidiaries of uniQure (uniQure, uniQure IP and such subsidiaries  
are hereinafter collectively referred to as “Borrower”) and Hercules Technology Growth Capital, Inc. (“Lender”) (the “Agreement”)

In connection with the above referenced Agreement, Borrower hereby authorizes the Lender to initiate debit entries for the periodic payments due under the Agreement to t Borrower’s account indicated below. Borrower authorizes the depository institution named below to debit to such account.

DEPOSITORY NAME	BRANCH
CITY	STATE AND ZIP CODE
TRANSIT/ABA NUMBER	ACCOUNT NUMBER

This authority will remain in full force and effect so long as any amounts are due under the Agreement.

\_\_\_\_\_  
(Borrower)(Please Print)  
By: \_\_\_\_\_  
Date: \_\_\_\_\_



## WARRANT AGREEMENT

To subscribe for Warrant Shares in the share capital of

## UNIQUE B.V.

Dated as of September 20, 2013 (the “Effective Date”)

WHEREAS, uniQure Biopharma B.V., a limited liability company organized under the laws of The Netherlands, has entered into a Loan and Security Agreement on or about 11 June 2013 (the “Loan Agreement”) with Hercules Technology Growth Capital, Inc., a corporation organized under the laws of the State of Maryland, USA (the “Warrantholder”);

WHEREAS, uniQure B.V., a limited liability company organized under the laws of The Netherlands (the “Company”), is the sole shareholder of uniQure Biopharma B.V.;

WHEREAS, the Company desires to grant to Warrantholder, in consideration for, among other things, the financial accommodations provided for in the Loan Agreement, the right to subscribe for Warrant Shares (as defined below) pursuant to this Warrant Agreement (this “Agreement”);

NOW, THEREFORE, in consideration of the Warrantholder executing the Loan Agreement and providing the financial accommodations contemplated therein, and in consideration of the mutual covenants and agreements contained herein, the Company and Warrantholder agree as follows:

## SECTION 1. DEFINITIONS AND INTERPRETATION

As used herein, the following terms shall have the following meanings:

“Company” means uniQure B.V., a limited liability company organized under the laws of The Netherlands, and any successor or surviving entity that assumes the obligations of the Company under this Agreement.

“Articles” means the Company’s articles of association, as may be amended and in effect from time to time.

“Ordinary Shares” means ordinary shares Class A (*gewone aandelen A*) in the issued share capital of the Company with a nominal value of EUR 0.01 each, or after an Initial Public Offering, such ordinary shares in the issued share capital of the Company as shall be issued in such Initial Public Offering.

“Exercise Price” shall mean US\$2.69 per Warrant Share.

“Initial Public Offering” means the initial underwritten public offering of the Company’s Ordinary Shares pursuant to a registration statement under the US Securities Act of 1922 as amended, or any successor statute, or pursuant to the laws of any non-US jurisdiction, which public offering has been declared effective by the SEC or has otherwise been consummated in accordance with the laws of such non-US jurisdiction.

“Merger Event” means any transaction or series of related transactions involving: (i) the sale, lease, exclusive license, or other disposition of all or substantially all of the assets of the Company, (ii) the merger or consolidation of the Company into or with another entity (other than a merger or consolidation effected exclusively to change the Company’s domicile), or any other corporate reorganization, in which the shareholders of the Company in their capacity as such immediately prior to such merger, consolidation or reorganization, own less than a majority of the Company’s (or the surviving or successor entity’s) outstanding voting power immediately after such merger, consolidation or reorganization (or, if such Company shareholders beneficially own a

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majority of the outstanding voting power of the surviving or successor entity as of immediately after such merger, consolidation or reorganization, such surviving or successor entity is not the Company); or (iii) any sale or other transfer by the shareholders of the Company of shares representing at least a majority of the Company’s then-total outstanding combined voting power. For the avoidance of doubt, the envisaged conversion of the legal form of the Company in connection with the Initial Public Offering does not qualify as a Merger Event.

“Warrant Shares” shall mean the Ordinary Shares.

“Shareholders Agreements” means the Class A Shareholders Agreement relating to the Company dated 19 April 2012 between the Company and the Investors (as defined therein) and the Class B Shareholders Agreement relating to the Company dated 19 April 2012 between the Company, the Stichting Administratiekantoor uniQure B.V. and the Investors (as defined therein).

“Subscription Price” means, with respect to any exercise of this Agreement, an amount equal to the Exercise Price as of the relevant time multiplied by the number of Warrant Shares subscribed for under this Agreement pursuant to such exercise.

## SECTION 2. GRANT OF THE RIGHT TO SUBSCRIBE FOR WARRANT SHARES.

(a) Grant of Warrants. For value received by its subsidiary uniQure Biopharma B.V., the Company hereby issues warrants (“Warrants”) to the Warrantholder, upon the terms and subject to the conditions of this Agreement. The Warrantholder shall have the number of Warrants equal to the number of Warrant Shares the Warrantholder may subscribe to as calculated in accordance with Section 2(b) below, each Warrant comprising the right and not the obligation to subscribe in cash for one Warrant Share at a subscription price per share equal to the Exercise Price (as defined below).

(b) Number of Shares. This Agreement shall be exercisable for 185,873 Warrant Shares, subject to adjustment thereafter from time to time in accordance with the provisions of this Agreement.

## SECTION 3. TERM OF THE AGREEMENT.

Except as otherwise provided for herein, the term of this Agreement and the right to subscribe for Warrant Shares as granted herein shall commence on the Effective Date Any and all Warrants which remain outstanding on the earlier of (i) ten (10) years from the Effective Date; or (ii) five (5) years after the Initial Public Offering, shall be immediately cancelled and the Warrantholder shall have no further rights under those Warrants as of such time.

#### SECTION 4. EXERCISE OF WARRANTS.

(a) Exercise. The subscription rights set forth in this Agreement are exercisable by the Warrantholder, in whole or in part, at any time, or from time to time, prior to the expiration of the term set forth in Section 3, by tendering to the Company at its principal office a notice of exercise in the form attached hereto as Exhibit I (the “Notice of Exercise”), duly completed and executed. The Company shall procure that promptly upon receipt of the Notice of Exercise, and in no event later than five (5) business days thereafter, any Warrant Shares to be issued to the Warrantholder upon the valid exercise of any Warrants shall be issued to the Warrantholder and, if the issuance takes place prior to any Initial Public Offering, that such Warrantholder is entered in the shareholders register of the Company as the holder of those Warrant Shares. If the issuance of

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Warrant Shares takes place after any Initial Public Offering, the Warrant Shares shall be delivered in book entry form to a securities account specified by the Warrantholder.

(b) Set-off. Parties hereto, including solely for purposes of this Section 4 (b) uniQure Biopharma B.V., hereby agree that, for as long as the Warrantholder also acts as Lender under the Loan Agreement, payment of (a portion of) the Subscription Price may be set off against any outstanding amounts payable by uniQure Biopharma B.V. under the Loan Agreement.

(c) Amendment Articles. The Company agrees that, if and when — at any time- an amendment of the Company’s Articles, including, but not limited to, an increase in the authorized capital of issuable shares, is necessary in order to allow for a sufficient number of Warrant Shares to be issuable upon the exercise of Warrants, then it will procure that any and all members of its group will, vote in favor of the necessary amendment(s) of the Company’s Articles and take any such further action as may necessary or appropriate in order to allow the Company to create the relevant Warrant Shares.

(d) Partial exercise. Upon partial exercise of the Warrants, the Company shall promptly register in the warrant registry the remaining number of Warrant Shares issuable hereunder.

#### SECTION 5. NO RIGHTS AS SHAREHOLDER.

This Agreement does not entitle the Warrantholder to any voting rights or other rights as a shareholder/ of the Company prior to the exercise of this Agreement.

#### SECTION 6. WARRANTHOLDER REGISTRY.

The Company shall maintain a registry showing the name and address of the Warrantholder of this Agreement, the number of Warrants, which at any time are outstanding, as well as the Exercise Price and scheduled expiry of such Warrants. Warrantholder’s initial address, for purposes of such registry, is set forth in Section 11(d) below. Warrantholder may change such address by giving written notice of such changed address to the Company in accordance with the requirements of Section 11(d).

#### SECTION 7. ADJUSTMENT RIGHTS.

The Exercise Price and the number of Warrant Shares issuable hereunder are subject to adjustment, as follows, provided however that no adjustment shall be made such that, on exercise Warrant Shares would be issued at a discount to their nominal value:

(a) Merger Event. If at any time there shall be a Merger Event as referred in Section 1 under (ii) of the definition of Merger Event, then, as a part of such Merger Event, appropriate adjustments shall be made so that the Warrantholder shall thereafter be entitled to receive, upon exercise of this Agreement, the number of shares, or other securities or monetary compensation (“Reference Property”), that the Warrantholder would have received in connection with such Merger Event if Warrantholder had exercised the Warrants immediately prior to the Merger Event. In any such case, appropriate adjustments (as determined in good faith by the Company’s Board of Directors) shall be made in the application of the provisions of this Agreement with respect to the rights and interests of the Warrantholder after the Merger Event to the end that the provisions of this Agreement (including adjustments of the Exercise Price and adjustments to ensure that the provisions of this Section 7 shall thereafter be applicable, as nearly as possible, to the subscription rights under this Agreement in relation to any Reference Property thereafter acquirable upon exercise of such subscription rights) shall continue to be applicable in their entirety, and to the greatest extent possible. Without limiting the foregoing, in connection with any Merger Event, upon the closing thereof, the successor or surviving entity shall assume

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the obligations of this Agreement; provided that if the Reference Property includes shares or other securities and assets of an entity other than the successor or purchasing company, as the case may be, in such Merger Event, then such other entity shall assume the obligations under this Agreement and any such assumption shall contain such additional provisions to protect the interests of the Warrantholder as reasonably necessary by reason of the foregoing (as determined in good faith by the Company’s Board of Directors). The provisions of this Section 7(a) shall similarly apply to successive Merger Events.

(b) Reclassification of Shares. Except for Merger Events subject to Section 7(a), and subject to Section 7(f), if the Company at any time shall, by combination, reclassification, exchange or subdivision of shares or otherwise, change any of the shares as to which subscription rights under this Agreement exist into the same or a different number of securities of any other class or classes, this Agreement shall thereafter represent the right to acquire such number and kind of securities as would have been issuable as the result of such change with respect to the securities which were subject to the purchase rights under this Agreement immediately prior to such combination, reclassification, exchange, subdivision or other change. The provisions of this Section 7(b) shall similarly apply to successive combination, reclassification, exchange, subdivision or other change.

(c) Subdivision or Combination of Shares. If the Company at any time shall combine or subdivide its Warrant Shares, (i) in the case of a subdivision, the Exercise Price shall be proportionately decreased, or (ii) in the case of a combination, the Exercise Price shall be proportionately increased.

(d) Shares Dividends. If the Company at any time while Warrants are outstanding and unexpired shall:

(i) pay a dividend to the Warrant Shares in the form of Warrant Shares (including any share premium account and, for the avoidance of doubt, a dividend that permits the recipient to elect between cash and Warrant Shares), then the Exercise Price shall be adjusted, from and after the date of determination of shareholders entitled to receive such dividend or distribution, to that price determined by multiplying the Exercise Price in effect immediately prior to such date of determination by a fraction (A) the numerator of which shall be the total number of Warrant Shares outstanding immediately prior to such dividend or distribution, and (B) the denominator of which shall be the total number of Warrant Shares outstanding immediately after such dividend or distribution; or

(ii) make any other distribution with respect to Warrant Shares (or shares into which the Warrant Shares are convertible and including any distribution that permits the recipient to elect between cash and Warrant Shares), except any distribution specifically provided for in any other clause of this Section 7, then, in each such case, provision shall be made by the Company such that the Warrantholder shall receive upon exercise or conversion of this Warrant a proportionate share of any such distribution as though it were the holder of the Warrant Shares (or other Shares for which the Warrant Shares is convertible) as of the record date fixed for the determination of Shareholders of the Company entitled to receive such distribution.

(e) Antidilution Rights. All Warrant Shares to be issued hereunder shall rank pari passu and form one class with the Warrant Shares issued and outstanding on the relevant date of exercise of such Warrants. Additional antidilution rights applicable to the Warrant Shares issuable hereunder are as set forth in the Articles and/or such other agreements entered into by shareholders

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of the Company and shall be applicable with respect to the Warrant Shares issuable hereunder. The Company shall promptly provide the Warrantholder with any restatement, amendment, modification or waiver of the Articles and/or the Shareholders Agreements; provided, that no such amendment, modification or waiver shall impair or reduce the antidilution rights applicable to the Warrant Shares as of the date hereof unless such amendment, modification or waiver affects the rights of Warrantholder with respect to the Warrant Shares in the same manner as it affects all other holders of Warrant Shares. The Company shall provide Warrantholder with prior written notice of any issuance of shares in the share capital of the Company or other equity security to occur after the Effective Date of this Agreement, which notice shall include (a) the price at which such shares or security is to be issued, (b) the number of shares to be issued, and (c) such other information as necessary for Warrantholder to determine if a dilutive event has occurred. For the avoidance of doubt, there shall be no duplicate anti-dilution adjustment pursuant to this subsection (e), the forgoing subsection (d) and the Articles.

(f) "Pay to Play" Rights. In the event that any "pay to play" terms or conditions (i.e. terms or conditions that require a holder of the Warrant Shares to purchase securities in a future round of equity financing or else lose the benefit of anti-dilution protections applicable to Warrant Shares or have such Warrant Shares automatically convert into Ordinary Shares or another class or series of shares) in the Articles and/or the Shareholders Agreements are triggered in connection with any Equity Round (a "Trigger Event"), then, in each such event, the subscription rights under this Agreement shall automatically adjust to provide the Warrantholder, upon the later exercise hereof, with the same securities and/or rights that the Warrantholder would have received had the Warrantholder (i) exercised this Warrant prior to such Trigger Event, and (ii) participated in the applicable equity financing in an amount sufficient to be deemed to have fully participated for purposes of such "pay to play" provision.

(g) Notice of Adjustments. If: (i) the Company shall declare any dividend or distribution upon its shares, whether in shares, cash, property or other securities; (ii) the Company offer for subscription pro rata to the holders of the outstanding Warrant Shares any additional shares or other securities of any class or other rights to subscribe for or purchase same; (iii) there shall be any Merger Event; (iv) there shall be an Initial Public Offering; (v) the Company shall sell, lease, license or otherwise transfer all or substantially all of its assets; or (vi) there shall be any voluntary dissolution, liquidation or winding up of the Company; then, in connection with each such event, the Company shall send to the Warrantholder: (A) at least ten (10) days' prior written notice of the date on which the books of the Company shall close or a record shall be taken for such dividend, distribution, subscription rights (specifying the date on which the holders of Warrant Shares shall be entitled thereto) or for determining rights to vote in respect of such Merger Event, dissolution, liquidation or winding up; (B) in the case of any such Merger Event, sale, lease, license or other transfer of all or substantially all assets, dissolution, liquidation or winding up, at least ten (10) days' prior written notice of the date when the same shall take place (and specifying the date on which the holders of Warrant Shares shall be entitled to exchange their Warrant Shares for securities or other property deliverable upon such Merger Event, dissolution, liquidation or winding up); and (C) in the case of an Initial Public Offering, the Company shall give the Warrantholder at least ten (10) days' written notice prior to the effective date thereof.

Each such written notice shall set forth, in reasonable detail, (i) the event requiring the notice, and (ii) if any adjustment is required to be made, (A) the amount of such adjustment, (B) the method by which such adjustment was calculated, (C) the adjusted Exercise Price (if the Exercise Price has been adjusted), and (D) the number of shares subject to subscription hereunder after giving effect to such adjustment, and shall be given by reputable overnight courier with all charges prepaid, addressed to the Warrantholder at the address for Warrantholder set forth in the registry referred to in Section 6.

(h) Timely Notice. Failure to timely provide such notice required by subsection (g) above shall entitle Warrantholder to retain the benefit of the applicable notice period

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notwithstanding anything to the contrary contained in any insufficient notice received by Warrantholder. For purposes of this subsection (h), and notwithstanding anything to the contrary in Section 11(d), the notice period shall begin on the date Warrantholder actually receives a written notice containing all the information required to be provided in such subsection (g).

## **SECTION 8. REPRESENTATIONS, WARRANTIES AND COVENANTS OF THE COMPANY.**

(a) Articles. The Company has made available to the Warrantholder true, correct and complete copies of the current Articles and the Shareholders Agreements.

(b) Due Authority. The execution by the Company of this Agreement and the performance of all obligations of the Company hereunder, including the issuance of Warrants to Warrantholder, have been duly authorized by all necessary corporate action on the part of the Company and any member of its group. This Agreement: (1) does not violate the Articles; (2) does not contravene any law or governmental rule, regulation or order applicable to it; and (3) does not and will not contravene any provision of, or constitute a default under, any material agreement or instrument binding upon it or a member of its groups or constitute a material default or termination event (however described) under any such agreement or instrument to which it is a party or by which it is bound at the date of this Agreement. This Agreement constitutes a legal, valid and binding agreement of the Company, enforceable in accordance with its terms.

(c) Consents and Approvals. No consent or approval of, giving of notice to, registration with, or taking of any other action in respect of any governmental authority is required with respect to the execution and performance by the Company of its obligations under this Agreement.

(d) Issued Securities.

(i) The authorized capital of the Company consists of (A) 200,000,000 shares, of which 60,957,675 shares are issued and outstanding.

(ii) The Company has reserved 8,439,126 for issuance under its Shares Option Plan(s), under which 8,428,167 options are outstanding. Other than the Shares Option Plan(s) and the Convertible Note Amendment Agreement, dated March 1, 2013, there are no other options, warrants, conversion privileges or other rights presently outstanding to subscribe for or purchase or otherwise acquire any authorized but unissued shares in the share capital of the Company or other securities of the Company. The Company has no outstanding loans to any employee, officer or director of the Company, and the Company agrees not to enter into any such loan or otherwise guarantee the payment of any loan made to an employee, officer or director by a third party.

(e) Insurance. The Company has in full force and effect insurance policies, with extended coverage, insuring the Company and its property and business against such losses and risks, and in such amounts, as are customary for corporations engaged in a similar business and similarly situated and as otherwise may be required pursuant to the terms of any other contract or agreement.

(f) Information Rights. During the term of this Warrant, Warrantholder shall be entitled to the information rights contained in Section 7.1 of the Loan Agreement, and Section 7.1 of the Loan Agreement is hereby incorporated into this Agreement by this reference as though fully set forth herein, provided, however, that the Company shall not be required to deliver a

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Compliance Certificate once all Indebtedness (as defined in the Loan Agreement) owed by the uniQure Biopharma B.V. to Warrantholder has been repaid.

#### **SECTION 9. REPRESENTATIONS AND COVENANTS OF THE WARRANTHOLDER.**

This Agreement has been entered into by the Company in reliance upon the following representations and covenants of the Warrantholder:

(a) Financial Risk. The Warrantholder has such knowledge and experience in financial and business matters as to be capable of evaluating the merits and risks of its investment, and has the ability to bear the economic risks of its investment.

(b) The execution by the Warrantholder of this Agreement and the performance of all obligations of the Warrantholder hereunder have been duly authorized by all necessary corporate action on the part of the Warrantholder and any member of its group. This Agreement constitutes a legal, valid and binding agreement of the Warrantholder, enforceable in accordance with its terms.

#### **SECTION 10. TRANSFERS**

The Warrantholder may transfer its rights or obligations under this Agreement, with the Company's prior written consent, which shall not be unreasonably withheld, by executing a deed of transfer in the form attached hereto as Exhibit II (the "Transfer Deed"). The duly executed Transfer Deed should promptly be issued to the Company to enable the Company to register the transfer in the warrant registry. Any transfer of Warrant Shares issued upon the exercise of Warrants shall be subject to the Articles and, if applicable, the Shareholders Agreements and the transfer restrictions set out therein for transfer of shares.

#### **SECTION 11. MISCELLANEOUS.**

(a) Effective Date. The provisions of this Agreement shall be construed and shall be given effect in all respects as if it had been executed and delivered by the Company on the date hereof.

(b) Remedies. The Parties waive their rights to (a) rescind or to seek to rescind (*ontbinden*) the Agreement, including, without limitation, the rights and defences contemplated by section 6:265 of the Dutch Civil Code performed under or pursuant to the Agreement, (b) suspend (*opschorten*) any of its obligations under this Agreement pursuant to section 6:52, 6:262 or 6:263 of the Dutch Civil Code or on any other ground and (ii) nullify (*vernietigen*) this Agreement pursuant to section 6:228 of the Dutch Civil Code or on any other ground.

(c) Severability. In the event any one or more of the provisions of this Agreement shall for any reason be held invalid, illegal or unenforceable, the remaining provisions of this Agreement shall be unimpaired, and the invalid, illegal or unenforceable provision shall be replaced by a mutually acceptable valid, legal and enforceable provision, which comes closest to the intention of the parties underlying the invalid, illegal or unenforceable provision.

(d) Notices. Except as otherwise provided herein, any notice, demand, request, consent, approval, declaration, service of process or other communication that is required, contemplated, or permitted under this Agreement or with respect to the subject matter hereof shall be in writing, and shall be deemed to have been validly served, given, delivered, and received upon the earlier of: (i) the day of transmission by facsimile or hand delivery if transmission or

delivery occurs on a business day at or before 5:00 pm in the time zone of the recipient, or, if transmission or delivery occurs on a non-business day or after such time, the first business day thereafter, or the first business day after deposit with an overnight express service or overnight mail delivery service; or (ii) the third calendar day after deposit in the United States mails, with proper first class postage prepaid, and shall be addressed to the party to be notified as follows:

If to Warrantholder:

HERCULES TECHNOLOGY GROWTH CAPITAL, INC.  
Legal Department  
Attention: Chief Legal Officer and Manuel Henriquez  
400 Hamilton Avenue, Suite 310  
Palo Alto, CA 94301  
USA  
Facsimile: 650-473-9194  
Telephone: 650-289-3060

(i) If to the Company:

UNIQUE B.V.  
Attention: Chief Financial Officer  
Meibergdreef 61  
1105 BA Amsterdam  
The Netherlands  
Facsimile:  
Telephone:

or to such other address as each party may designate for itself by like notice.

(e) Entire Agreement; Amendments. This Agreement constitutes the entire agreement and understanding of the parties hereto in respect of the subject matter hereof, and supersede and replace in their entirety any prior proposals, term sheets, letters, negotiations or other documents or agreements, whether written or oral, with respect to the subject matter hereof (including Lender's proposal letter dated April 18, 2013). None of the terms of this Agreement may be amended except by a written instrument executed by each of the parties hereto.

(f) Headings. The various headings in this Agreement are inserted for convenience only and shall not affect the meaning or interpretation of this Agreement or any provisions hereof.

(g) Advice of Counsel. Each of the parties represents to each other party hereto that it has discussed (or had an opportunity to discuss) with its counsel this Agreement.

(h) No Strict Construction. The parties hereto have participated jointly in the negotiation and drafting of this Agreement. In the event an ambiguity or question of intent or interpretation arises, this Agreement shall be construed as if drafted jointly by the parties hereto and no presumption or burden of proof shall arise favoring or disfavoring any party by virtue of the authorship of any provisions of this Agreement.

(i) No Waiver. No omission or delay by Warrantholder at any time to enforce any right or remedy reserved to it, or to require performance of any of the terms, covenants or provisions hereof by the Company at any time designated, shall be a waiver of any such right or remedy to which Warrantholder is entitled, nor shall it in any way affect the right of Warrantholder to enforce such provisions thereafter.

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(j) Survival. All agreements, representations and warranties contained in this Agreement or in any document delivered pursuant hereto shall be for the benefit of Warrantholder and shall survive the execution and delivery of this Agreement and the expiration or other termination of this Agreement.

(k) Governing Law. This Agreement is governed by Dutch law.

(l) Enforcement. The courts (*rechtbank*) of Amsterdam, The Netherlands, subject to ordinary appeal (*hoger beroep*) and final appeal (*cassatie*), shall have exclusive jurisdiction to hear and determine any suit, action or proceeding and to settle any dispute arising out of or in connection with this Agreement (including a dispute regarding the existence, validity or termination of this Agreement) and, for such purposes, each of the Parties hereto irrevocably submits to the exclusive jurisdiction of such courts.

(m) Counterparts. This Agreement and any amendments, waivers, consents or supplements hereto may be executed in any number of counterparts, and by different parties hereto in separate counterparts, each of which when so delivered shall be deemed an original, but all of which counterparts shall constitute but one and the same instrument.

(n) Language. The official language of this Agreement and of all notices and other communications by a party or between the parties hereunder shall be in English.

IN WITNESS WHEREOF, the parties hereto have caused this Agreement to be executed by its officers thereunto duly authorized as of the Effective Date.

COMPANY:

UNIQUE B.V.

By: /s/PJ Morgan  
Name: PJ Morgan  
Title: CFO

WARRANTHOLDER:

HERCULES TECHNOLOGY GROWTH  
CAPITAL, INC.

By: /s/K. Nicholas Martitsch  
Name: K. Nicholas Martitsch  
Title: Associate General Counsel

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SOLELY FOR PURPOSES OF SECTION 4 (b):

UNIQUE BIOPHARMA B.V.

By: /s/PJ Morgan  
Name: PJ Morgan  
Title: CFO

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## EXHIBIT I

### NOTICE OF EXERCISE

To: [ ]

(1) This is a Warrant notice of exercise, Terms defined in the warrant agreement dated the [ ] day of [ ], [ ] (the “Agreement”) between [ ] and the Warrantholder (“Agreement”) shall have the same meaning in this exercise notice unless otherwise defined herein.

(2) The undersigned Warrantholder hereby exercises [ ] Warrants, pursuant to the terms of the Agreement, which gives us the right to acquire [\*] Warrant Shares upon payment of the Subscription Price.

(2) Please notify us within three Business Days when the deed of issue relating to the Warrant Shares to be issued will be executed and, if applicable, which notary will execute such deed of issue.

WARRANTHOLDER:

HERCULES TECHNOLOGY GROWTH  
CAPITAL, INC.

By: \_\_\_\_\_  
Name: \_\_\_\_\_  
Title: \_\_\_\_\_  
Date: \_\_\_\_\_

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## EXHIBIT II

### TRANSFER DEED

(To transfer the foregoing Agreement execute this form and supply required information.)

FOR VALUE RECEIVED, the foregoing Agreement and all rights evidenced thereby are hereby transferred to

\_\_\_\_\_  
(Please Print)

whose address is \_\_\_\_\_

Dated: \_\_\_\_\_  
Warrantholder's Signature: \_\_\_\_\_  
Warrantholder's Address: \_\_\_\_\_

Dated: \_\_\_\_\_  
Transferee's Signature: \_\_\_\_\_  
Transferee's Address: \_\_\_\_\_





Execution version




# Subscription Agreement

between

Chiesi Farmaceutici S.p.A.,  
as the Investor

and

uniQure B.V.,  
as the Company

  
pe  

## SUBSCRIPTION AGREEMENT

THIS SUBSCRIPTION AGREEMENT is made on 29 April 2013

BETWEEN:

- 1 **Chiesi Farmaceutici S.p.A.**, a company organized under the laws of Italy, with its offices at Via Palermo, 26/A, 43122 Parma, Italy (the "Investor"); and
- 2 **uniQure B.V.**, a private company with limited liability incorporated and existing under the laws of the Netherlands, having with its corporate seat at Amsterdam, the Netherlands and its registered address at Meibergdreef 61, 1105 BA Amsterdam Zuidoost, the Netherlands and registered with the trade register of the Chamber of Commerce for Amsterdam with registration number 54385229 (the "Company").

The Investor and the Company, hereinafter jointly referred to as the "Parties" and each of them individually as a "Party".

WHEREAS:

- (A) The Investor and uniQure biopharma B.V. (a wholly-owned subsidiary of the Company) intend to enter into (i) a commercialisation agreement for the sale of Glybera® in Europe and certain other additional specified territories (the "Commercialisation Agreement"), and (ii) a co-development and license agreement in respect of a Hemophilia B gene therapy programme for certain European and other additional specified territories (the "Co-Development and License Agreement").
- (B) Subject to the terms and conditions of this Agreement, the Parties have agreed that on Closing Date the Investor will subscribe for such number of new class C ordinary shares, with a nominal value of €0.01 each, in the capital of the Company as set out opposite the Investor's name in Schedule 1 (the "Investor Shares") for a subscription price of €2.52431 per Investor Share (the "Subscription Price").
- (C) Immediately following the Closing, the Investor Shares will represent not less than 8.75% of the share capital of the Company, including dilution arising from existing warrants and share options.
- (D) Before Closing, the Company shall obtain approval by the Company's shareholders meeting, in which it is resolved (i) to amend the Company's articles of association, (ii) to issue the Investor Shares to the Investor against the Subscription Price per Investor Share on the terms as set forth in this Agreement, and (iii) to exclude the pre-emptive rights of the existing shareholders of the Company in relation to such share issuance.

AGREE AS FOLLOWS:

### 1 Interpretation

- 1.1 Unless explicitly stated otherwise, the following terms shall have the following meaning (and grammatical variations of such terms shall have corresponding meanings) in this Agreement:

"Agreement" means this subscription agreement including the recitals and the Schedules thereto;



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"Closing Date" means the date on which the Closing occurs, which shall be on the third business day following the date of this Agreement or such later date that the Company shall reasonably determine, and in any event not later than 30 June 2013;

"Closing" means the consummation of the transactions set out in clause 2 in accordance with clause 4;

"Company" as defined above;

"Company Warranties" means the representations and warranties made by the Company to the Investor contained in Schedule 2;

"Encumbrance" means any right of pledge, mortgage, usufruct, retention of title or other security interest whatsoever and any arrest, charge, attachment, option or lien or any similar concept that limits free and unrestricted title and/or use, under any applicable law;

"Investor" as defined above;

"Investor Warranties" means the representations and warranties made by the Investor to the Company contained in Schedule 3;

"Investor Shares" has the meaning ascribed to it in recital (B);

"Notary" means Mr P.H.N. Quist or his substitute, civil law notary at the offices of Stibbe N.V. in Amsterdam, The Netherlands;

"Notary's Account" has the meaning ascribed to it in clause 4.2;

"Parties" as defined above;

"Schedule" means a schedule to this Agreement;

"Share Issue Deed" means the notarial share issue deed to be executed in connection with the effectuation of the issuance of the Investor Shares;

"Shares" means class A, class B or class C ordinary shares in the capital of the Company with a nominal value of €0.01 each, outstanding from time to time; and

"Subscription Price" has the meaning ascribed to it in recital (B).

1.2 In this Agreement, a reference to:

- 1.2.1 singular words shall include the plural and *vice versa* and words in a particular gender shall include all genders, unless the context requires otherwise;
- 1.2.2 the word "include" or "including" are used to indicate that the matters listed are not a complete enumeration of all matters covered, unless the contrary is specifically stated;
- 1.2.3 the words "hereof", "herein", "hereto" and "hereunder" and words of similar import shall refer to this Agreement as a whole and not to any particular provision thereof;
- 1.2.4 a clause or a schedule is a reference to a clause or schedule of the actual agreement, or, if specifically mentioned, to a clause to the Investor Warranties or the Company Warranties; and

1.2.5 a person includes a reference to a body corporate, association or partnership.

- 1.3 In this Agreement, clause headings are inserted for convenience purposes only. They shall not affect the construction or interpretation of this Agreement.
- 1.4 In case of conflict between or inconsistency of the provisions of the actual agreement and the contents of the Schedules, the provisions of the actual agreement shall prevail.
- 1.5 The English language used in this Agreement intends to describe Dutch legal concepts only and the consequences of the use of this language in English law or any other law shall be disregarded. In case of conflict between Dutch legal concepts mentioned between brackets and/or in italics in this Agreement and the English translation thereof as used in this Agreement, the Dutch text, and its meaning thereof under Dutch law, will prevail.

## **2 Subscription and Issue**

- 2.1 Subject to the terms and conditions of this Agreement, and in reliance on the Company Warranties, the Investor hereby agrees to subscribe for the Investor Shares on the Closing Date against payment of the Subscription Price per Investor Share in accordance with clause 4.
- 2.2 Subject to the terms and conditions of this Agreement, and in reliance on the Investor Warranties, the Company hereby agrees to issue the Investor Shares subscribed for by the Investor on the Closing Date against receipt of the aggregate Subscription Price for such Investor Shares in accordance with clause 4.

## **3 Conditions Precedent**

The obligations of (a) the Investor to subscribe for the Investor Shares in accordance with clause 2.1 above, and (b) of the Company to issue the Investor Shares in accordance with clause 2.2 above, shall each be subject to the following conditions precedent ("*opschortende voorwaarden*") (the "**Conditions Precedent**"):

- (a) the Investor and uniQure biopharma B.V. having entered into the Commercialization Agreement and such agreement having become unconditional in all respects; and
- (b) the Investor and uniQure biopharma B.V. having entered into the Co-Development and License Agreement and such agreement having become unconditional in all respects.

## **4 Closing**

- 4.1 Upon fulfilment, or waiver, of the Conditions Precedent, **Closing** shall take place on the Closing Date at the offices of the Notary, or at such other place the Parties may agree.
- 4.2 No later than two business days prior to the Closing Date, the Investor shall pay the aggregate Subscription Price for the Investor Shares it has subscribed for, to be paid in full, without any deductions or set-off, by wire transfer and in immediately available funds to the trust account of the office of the Notary, bank account number 69.64.62.672 in the name of Stibbe Derdengelden Notariaat with ING Bank N.V. (IBAN: NL79 INGB 0696 4626 72 and BIC INGBNL2A (the "**Notary's Account**").
- 4.3 At Closing, after having received the confirmation from the Notary that the Subscription Price for the Investor Shares that the Investor has subscribed for has been paid in full

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without any deductions or set-off in immediately available funds on the Notary's Account, the Investor and the Company shall execute the Share Issue Deed in order to effectuate the issuance of the Investor Shares.

5 **Warranties**

- 5.1 The Company represents and warrants to the Investor that each of the Company Warranties is true and correct on the date hereof and shall be true and correct on the Closing Date.
- 5.2 The Investor represents and warrants to the Company that in respect of the Investor each of the Investor Warranties is true and correct on the date hereof and shall be true and correct on the Closing Date.
- 5.3 In the event of a breach of any of the Company Warranties or the Investor Warranties, the defaulting Party shall pay an amount necessary to compensate the damages of the other Party to whom the warranties are given, which shall not include any consequential loss suffered by such Party.
- 5.4 The Parties understand and acknowledge that in relation to the subscription no prospectus shall be prepared nor made available, and that the Investor Shares are unlisted securities which shall not, at this time, be listed on any recognised investment exchange.

6 **Confidentiality**

Each Party undertakes not to disclose the existence of this Agreement or to divulge any part of its contents to any third party, other than as a necessary result of the exercise of any rights under this Agreement or if obliged to do so by applicable securities or other law or competent regulatory authorities, provided, however, that the Company may make a public announcement regarding the transactions contemplated by this Agreement in a form to be agreed with the Investor, such agreement not to be unreasonably withheld.

7 **Binding effect: assignment**

- 7.1 All the terms, provisions, representations, warranties, covenants and conditions of this Agreement shall only be binding upon and inure to the benefit of and be enforceable by the Parties hereto after this Agreement has been signed by all Parties.
- 7.2 Except as expressly set out otherwise, this Agreement and any rights and obligations of the Investor hereto cannot be assigned or delegated by the Investor to a third party without the prior written consent of the Company.

8 **Dissolution and partial invalidity**

- 8.1 Each of the Parties hereby waives the right, and each of the Parties accepts the same, to cancel (*opzeggen*), to dissolve or bring an action to dissolve this Agreement (*ontbinding*) and/or to annul or bring an action to annul this Agreement (*vernietiging*) or alter this Agreement on the basis of unforeseen circumstances (*onvoorziene omstandigheden*) or suspend (*opschorten*) any of the obligations assumed hereunder as from the moment of its execution.
- 8.2 In the event that one or more clauses of this Agreement or of the Schedules would appear to be non-binding, the other clauses of this Agreement and of the Schedules will continue to be effective. The Parties are obliged to replace the non-binding clauses with other



clauses that are binding, in such a way that the new clauses differ as little as possible from the non-binding clauses, taking into account the object and the purpose of this Agreement.

9 **Entire agreement**

This Agreement contains all arrangements which the Parties have made on the subject hereof and thereof. They shall take the place of all other (previous) arrangements and agreements, which any of the Parties have made or have entered into on the subject hereof and thereof.

10 **Expenses**

Each of the Parties hereto shall pay its own expenses incurred or to be incurred in connection with this Agreement.

11 **Notices**

11.1 As long as the Investor does not give notice to the Company of any other address, all announcements or notices by the Company to the Investor will be done in writing or by telefax (but in the case of a telefax immediately confirmed in writing) to the Investor's address mentioned in Schedule 1.

11.2 All announcements or notices by the Investor to the Company will be done in writing or by telefax (but in the case of a telefax immediately confirmed in writing) to:

uniQure B.V.  
Attn: the Executive Board  
P.O. Box 22506  
1100 DA Amsterdam  
The Netherlands  
Per fax: +31 20 566 9272

12 **Governing law and jurisdiction**

12.1 This Agreement and any non-contractual obligations arising there from or connected with it shall be governed by, and construed in accordance with, the laws of the Netherlands and the courts of Amsterdam, the Netherlands shall have exclusive jurisdiction in respect of any disputes relating to it.

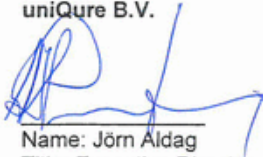
12.2 This Agreement may be executed in any number of counterparts and each of the executed counterparts, when duly exchanged or delivered, shall be deemed to be an original, but taken together, they shall constitute one instrument.

Thus executed on the day and year first above written.


[signature page to follow]

The block contains several handwritten signatures in blue ink. Above the signatures is a small rectangular stamp with the text "5963788\_1". The signatures appear to be initials or full names, some with a circular mark around them.

uniQure B.V.

1/A   
Name: Jörn Aldag  
Title: Executive Director

*J. Preustling*  
*VP, Business Development*  
Chiesi Farmaceutici S.p.A.

  
Name: Mr. Alberto Chiesi  
Title: President

uniQure B.V.

  
Name: Piers Morgan  
Title: Executive Director

Chiesi Farmaceutici S.p.A.

  
Name: Mr. Ugo Di Francesco  
Title: CEO



# SCHEDULE 1- INVESTOR

Investor	Number of Investor Shares	Aggregate Subscription Price
Chiesi Farmaceutici S.p.A.	5,546,070	€ 14,000,000

Unless the Investor gives notice to the Company of another address in accordance with clause 11 of the Agreement, all announcements or notices to the Investor shall be done in accordance with clause 11 of the Agreement to the following address:

Chiesi Farmaceutici S.p.A.  
Attn: CEO  
Via Palermo, 26/A,  
43122 Parma  
Italy  
Per fax: +39 0521 774468

  
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## SCHEDULE 2 – COMPANY WARRANTIES

- 1 The Company has been duly incorporated and is validly existing as a private company with limited liability under the laws of the Netherlands and has all requisite power to carry on its business as presently conducted.
- 2 The Company has not been dissolved and no resolution to dissolve the Company has been adopted. The Company has not been granted a moratorium of payment or been declared bankrupt.
- 3 The Company has full power and authority (corporate or otherwise) to enter into, execute, deliver and carry out the terms of the Agreement and to incur its obligations provided for herein, all of which have been duly authorised by all necessary corporate action and is not in violation of its articles of association or governing documents.
- 4 No consent, authorisation or approval of, filing with, notice to, or exemption by, any person or any governmental authority is required to authorise or is required in connection with the execution, delivery and performance by the Company of the Agreement, or is required as a condition to the validity or enforceability of the Agreement.
- 5 The Agreement constitutes legal and binding obligations for the Company, enforceable in accordance with its terms, except as such enforceability may be limited by applicable bankruptcy, insolvency, reorganisation or other similar laws affecting the enforcement of the creditors' rights generally or by general principles of equity.
- 6 The Investor Shares are duly authorised and, when issued and paid for in accordance with the Agreement, (i) the Investor Shares will be duly and validly issued, fully paid and nonassessable, (ii) the Investor Shares will form a new class of ordinary shares, which shall have the same dividend and voting rights as all of the other Shares (iii) issuance of the Investor Shares will not be subject to pre-emptive rights, (iv) the Investor will acquire full ownership of the Investor Shares, free and clear of any Encumbrance, and (v) immediately following the Closing, the Investor Shares will represent not less than 8.75% of the share capital of the Company, including dilution arising from existing warrants and share options.



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### SCHEDULE 3 – INVESTOR WARRANTIES

- 1 The Investor has been duly incorporated and is validly existing as a company under the laws of Italy.
- 2 The Investor has not been dissolved and no resolution to dissolve the Investor has been adopted. The Investor has not been granted a moratorium of payment or been declared bankrupt.
- 3 The Investor has full power and authority (corporate or otherwise) to enter into, execute, deliver and carry out the terms of the Agreement and to incur its obligations provided for herein, all of which have been duly authorised by all necessary corporate action and is not in violation of its articles of association or governing documents.
- 4 Except as specifically set forth in the Agreement, no consent, authorisation or approval of, filing with, notice to, or exemption by, any person or any governmental authority is required to authorise or is required in connection with the execution, delivery and performance by the Investor of the Agreement, or is required as a condition to the validity or enforceability of the Agreement.
- 5 The Agreement constitutes legal and binding obligations for the Investor, enforceable in accordance with its terms, except as such enforceability may be limited by applicable bankruptcy, insolvency, reorganisation or other similar laws affecting the enforcement of the creditors' rights generally or by general principles of equity.
- 6 The Investor is not a person located in the United States and is eligible to participate in an "offshore transaction", as defined in Regulation S under the US Securities Act of 1933, as amended (the "Securities Act"), conducted in accordance with Regulation S under the Securities Act and the Investor Shares were not offered to it by means of "directed selling efforts" as defined in Regulation S promulgated under the Securities Act.
- 7 The Investor is a qualified investor in the meaning of Directive 2003/71/EC of the European Parliament and of the European Council of 4 November 2003, as amended by Directive 2010/73/EU.
- 8 The Investor understands that the Investor Shares are being offered in a transaction not involving any (public) offering in the United States within the meaning of the Securities Act and that the Investor Shares are not being and will not be registered under the Securities Act or under any securities laws of any state or other jurisdiction of the United States and may not be offered, sold, resold, taken up, exercised, renounced, transferred or delivered, directly or indirectly, within the United States except pursuant to an applicable exemption from the registration requirements of the Securities Act and in compliance with any applicable securities laws of any state or other jurisdiction of the United States.
- 9 The Investor understands that nothing in the Agreement or any other information presented to the Investor in connection with the subscription and sale of the Investor Shares constitutes legal, tax or investment advice.
- 10 The Investor understands that the Investor Shares will be "restricted securities" within the meaning of Rule 144(a)(3) under the Securities Act and it agrees that for so long as such securities are "restricted securities" (as so defined), they may not be deposited into any unrestricted depository facility established or maintained by any depository bank.

  
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- 11 As long as the Investor Shares are "restricted securities" within the meaning of Rule 144(a)(3) under the Securities Act, the Investor will not reoffer, resell, pledge or otherwise transfer the Investor Shares, except in an offshore transaction in accordance with Rule 903 or Rule 904 of Regulation S under the Securities Act (which, for the avoidance of doubt, includes a sale over Euronext Amsterdam) or some other available exemption from the registration requirements of the Securities Act and in accordance with any applicable securities laws of any state or other jurisdiction of the United States.

  
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## uniQure 2012 STOCK OPTION PLAN

This stock option plan (the “Plan”) sets forth the rules on the basis of which options can be granted to employees of uniQure B.V. (“uniQure”) to acquire shares in the capital of uniQure. The rationale is that these employees are expected to contribute to the future growth and success of uniQure. This Plan may be amended from time to time.

Employees have no contractual right to participate in the Plan, which will be at the sole discretion of the Board. The level of any participation will be set by the Board, having reference to the Supervisory Board of the Company.

The purpose of the Plan is to incentivise and reward employees in the performance of their roles, to align their interests with those of the Company in respect of sustainable value growth, to secure their services and to provide security in the event of a future change in control.

### Article 1 - Definitions

In this Plan, except where inconsistent with the subject or context, words and expressions below shall have the following meanings:

Board	The Company’s board of management (het bestuur).
Company	uniQure B.V.
Control	Directly or indirectly holding or controlling 50% or more of the Shares in the outstanding capital of the Company, or being able to exercise or otherwise direct 50% or more of the votes in the general meeting of the Company.
Member of the Group	The Company, its subsidiaries from time to time, and any other company which is associated with the Company and is designated by the Board as a Member of the Group.
Date of Grant	The date on which the Company grants, or is deemed to have granted, one or more Options to a Participant in accordance with the provisions of this Plan.
Depository Receipt	A Depository Receipt for one B ordinary share in the Company.
Economic Value	The value of a Depository Receipt which shall be determined as being equal to the price of uniQure ordinary shares B based on the pre-money value attributed to such shares at the most recent previous financing round.
Eligible Employee	Means any person who is an employee of a Member of the Group and who is nominated by the Board to participate in the Plan.
Grant	The grant of one or more Options to a Participant in accordance with the provisions of this Plan.

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Grant Letter	The letter on the format attached hereto as <u>Schedule I</u> evidencing the grant of Options subject to the terms and conditions of this Plan, indicating the number of Depository Receipts that may be acquired, the Option Exercise Price, the Date of the Grant, and a copy of the latest version of this Plan.
Option Exercise Price	The amount which will have to be paid for the acquisition of a Depository Receipt upon the exercise of the Option, calculated in accordance with the provisions in this Plan.
Option	The right to acquire one Depository Receipt in the Company for the Option Exercise Price during the Option Period, in accordance with the provisions of this Plan.
Option Period	The period specified in Article 3.
Participant	An Eligible Employee to whom an Option has been granted.
Plan	The Plan in its present form or as from time to time amended in accordance with the provisions hereof.
Supervisory Board	The Company’s board of supervisory directors ( <i>raad van commissarissen</i> )

### Article 2 - Grant of Options

2.1 Subject to the terms and conditions set forth in this Plan the Board may, after having received authorisation on its proposal thereto from the Supervisory Board, decide that the Company shall grant a number of Options to any Eligible Employee.

2.2 Options may be granted:

- (i) at a date within the first month of employment of the Eligible Employee with a Member of the Group;
- (ii) at a date within four weeks after publication of the annual account by the Company;
- (iii) at any other event to be specified by the Board.

2.3 The Option Exercise Price shall be the Economic Value at the Date of Grant.

- 2.4 The granting of Options will be at the discretion of the Board in accordance with section 2.1, and will not, in itself, give the Participant any right to acquire further Options.
- 2.5 Options will be granted by means of a Grant Letter. The Options will only be considered as granted and accepted if the Participant has returned a copy of the Grant Letter within 30 days to the Company, bearing the Participant's signature evidencing his/her acceptance of the Options granted under the terms and conditions of this Plan.
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- 2.6 If the Option is not accepted in accordance with Article 2.5, the Option will be deemed to be cancelled and shall cease to exist.

### **Article 3 - Option Period**

- 3.1 The Option Period commences at the Date of Grant and will last ten years, unless otherwise provided in this Plan.
- 3.2 Options that have not been exercised in accordance with Article 4 will lapse automatically after expiration of the Option Period.

### **Article 4 - Vesting and exercise of the Option**

- 4.1 The Options granted shall be exercisable if and insofar the Options are vested and the conditions of Article 4.4 are fulfilled. Vesting will occur according to the following exercise schedule:
- (a) At the first anniversary of the Grant of the Options: 1/3 of the Options initially granted vest.
  - (b) Following the first anniversary of the date of grant, the remaining options (representing 2/3 of the grant) shall vest pro rata on a straight line basis over the second and third years following the date of grant.
- 4.2 In addition to Article 4.1, vesting of the Options will occur on the moment a person (or a group of persons acting in concert) obtains Control of the Company.
- 4.3 The Board may in its sole and absolute discretion deviate from the aforementioned exercise schedule, though only to the benefit of the Participant.
- 4.4 Options are exercised by means of:
- a written exercise statement to that effect by the Participant on the format attached hereto as Schedule 2, addressed to the Company; and
  - payment of the Option Exercise Price by the Participant to the Company.
- The exercise statement must mention the Date(s) of Grant and the number of Options exercised.
- 4.5 The exercise statement shall be signed by the Participant and be accompanied by the payment of the aggregate Option Exercise Price into a bank account of the Company mentioned on the exercise statement.
- 4.6 Any wage tax, income tax or social security premiums due in respect of the Grant of the Options under this Plan including but not limited to, the exercise of the Options and the sale of the Depositary Receipts derived from such exercise, will be for the account of the Participant.
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- 4.7 Any Option exercised, shall cease to exist as from the moment the exercise statement referred to in Article 4.5 of this Plan, and payment of the Option Exercise Price have been received by the Company.

### **Article 5 - Issue of Shares**

- 5.1 Within 15 days after receipt of the exercise statement mentioned in Article 4.5 by the Company and provided that the Option Exercise Price due has been received by the Company, the Company shall issue or transfer the Shares to the Participant, and the Participant shall accept the number of Shares indicated on the exercise statement.
- 5.2 All costs connected with the abovementioned issue or transfer of Depositary Receipts to the Participant shall be borne by the Company.

### **Article 6 - Adjustments**

- 6.1 In case of a dilution (*verwatering*) of the Share capital of the Company, for instance as a result of the issuance of Shares charged against any reserve of the Company, the Option Exercise Price and/or the number of Options granted may be adjusted or corrected accordingly by the Board in such way (including retrospective adjustments) as the Board considers appropriate. The same applies to any consolidation or share split.
- 6.2 a. There shall be an acceleration of vesting, and all outstanding options shall be deemed to have vested as at such date in case of (i) a change of Control when such a change of Control first occurs or, (ii) if earlier, at the date that the stockholders of the Company approve an agreement to merge or otherwise dispose of the Company or (substantially all of) its business to another party, as a result of which the other party would exercise control; or (iii) at the date of a decision by the Supervisory Board of the Company to list the Company on a Recognised Investment Exchange.
- b. In case all or the majority of Shares in the capital of the Company are being acquired by another legal entity through a reorganisation or acquisition, the Company may determine that upon completion of such reorganisation or acquisition, the Options shall instead bear the right to acquire shares in the capital of that legal entity according to a ratio to be determined by the Board in its sole discretion. This ratio will be based on the fair market value of the shares involved in the reorganisation or acquisition.

- 6.3 Notwithstanding the above, the Board can determine in its sole discretion that amendments will be made to this Plan to reasonably provide for any changes in the circumstances other than those mentioned in this article 6, including but not limited to exchange of Shares with another company and restructuring of the share capital of the Company.
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#### **Article 7 - Restrictions**

- 7.1 By signing the Grant Letter, the Participant accepts and shall comply in full with all obligations arising out of the Plan.
- 7.2 If and to the extent that a Participant fails to comply in a timely manner with any obligation arising out of the Plan, the signed Grant Letter irrevocably authorises the Company to execute any actions, deeds and the like and to act on behalf of the Participant to ensure compliance with all of the obligations described above. The Company shall not exercise this authority for the purpose of transferring Shares unless and until the Option Exercise Price due has been deposited.

#### **Article 8 - Termination of employment**

- 8.1 Unless otherwise decided by the Board, the Option shall lapse in the event the Participant's employment with the Company is terminated for cause. The Option will lapse on the date of such termination, or notice of such termination has been given.
- 8.2 In the event the Participant's employment with the Company terminates for any reason other than as set out in 8.1 above, the Participant, or his heirs in the case of death, shall be entitled to exercise the Options, only to the extent vested, for a period of six months after the date of employment termination.
- 8.3 Unvested Options automatically lapse at the date of employment termination.

#### **Article 9 - Other conditions**

- 9.1 The Options may not be transferred, pledged or encumbered.
- 9.2 The Board may, at its discretion, make adjustments, modifications or alternative arrangements to this Plan, provided that it shall make a reasonable effort to procure that such adjustments, modifications or alternative arrangements to this Plan are not to the (material) detriment of the Participant.
- 9.3 The Plan will not be considered as part of any employment agreement or other agreement in force between the Participant and the Company or a Member of the Group and does not grant the Participant any rights towards the Company or a Member of the Group other than the rights which have been laid down in the Plan and the Grant Letter. The Grant of Options does not qualify as an employment condition.
- 9.4 If the Company grants an Option which is inconsistent with the Plan, the Option will be limited and will take effect from the Date of Grant on a basis consistent with the Plan.
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#### **Article 10 - Cash alternative**

- 10.1 The Board may, in its sole discretion, determine not to procure the transfer or issue of Depositary Receipts to a Participant who exercises his Option, but instead to pay him a cash amount equal to the amount by which the Economic Value of the Depositary Receipts in respect of which the Option is exercised on the Option exercise date exceeds the Option Exercise Price.

#### **Article 11 - Regulation of Conduct**

- 11.1 The Company shall adapt the Plan according to the applicable rules of conduct on securities ownership and transactions by Participants applicable at such time, and any Participant receiving Depositary Receipts pursuant to an exercise of Options shall abide by the Company's rules of conduct (including but not limited to the Internal Code on Inside Information), together with relevant regulations and legislation, with regard to any future disposals of such Depositary Receipts. The Participant is obliged to observe any and all applicable rules under Dutch law with regard to insider trading and market abuse, if applicable to the Participant.

#### **Article 12 - Dispute rules and applicable law**

- 12.1 This Option Plan shall be governed by the laws of the Netherlands.
- 12.2 All disputes arising in connection with this Plan shall be exclusively submitted to the jurisdiction of the competent court in Amsterdam, The Netherlands.
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uniQure

### **SCHEDULE 1: GRANT LETTER**

#### **GRANT LETTER**

This Grant Letter is made on the [·] day of [·], [····].

#### **BY AND BETWEEN:**

1. [·], residing at [·], hereinafter referred to as "the Participant";

and

2. uniQure B.V. (uniQure), a private limited liability company incorporated in the Netherlands having its official seat in Amsterdam, the Netherlands, hereinafter referred to as “the Company”;

Together hereinafter referred to as “**Parties**”;

**WHEREAS:**

- The Company has implemented the uniQure 2012 Stock Option Plan (hereinafter referred to as “the Plan”).
- The Company hereby wishes to offer the Participant certain Options as set out herein, pursuant to Article 2 of the Plan, under the terms and conditions of the Plan.

**THE PARTIES HERETO AGREE AS FOLLOWS:**

**Definitions and interpretation**

*Article 1*

Capitalised terms used and not otherwise defined in this Grant Letter shall have the meanings given thereto in the Plan. The terms and conditions of the Plan are applicable to this Grant Letter, and are deemed to be incorporated in this Grant Letter by reference. By signing this Grant Letter, the Participant accepts the terms and conditions of the Plan. A copy of the Plan is attached hereto.

Where the provisions of this Grant Letter deviate from or are in conflict with the provisions and/or purpose of the Plan, the provisions of the Plan shall prevail, save in respect of references to “Eligible Employee” and or “employee” which shall be deemed to include individuals holding office as STAK Board members, and references in Article 8 of the Plan shall be interpreted with respect to holding such office.

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**Grant of Options**

*Article 2*

Subject to the terms and conditions of the Plan and this Grant Letter, the Company hereby grants to the Participant [ · ] Options.

**Option Period**

*Article 3*

The Date of Grant of the Options is [·].

**Option Exercise Price**

*Article 4*

The Option Exercise Price is hereby established at [·] per Depositary Receipt, in accordance with section 2.3 of the Plan.

**Exercise**

*Article 5*

The Options can only be exercised if and to the extent the Options have vested, and are not expired or lapsed, all in accordance with Article 4 of the Plan, and subject to the other terms and conditions of the Plan.

**Non transferability**

*Article 6*

The Options granted to the Participant under this Grant Letter can not be transferred, pledged or encumbered in any way, either in full or in part. Breach of this article will cause the Option to lapse forthwith.

**Taxes, social security premiums**

*Article 7.a.*

Any wage or personal or corporate income tax, or social security premiums due in connection with the Options, including but not limited to any wage tax, income tax or social security premiums due in connection with the grant, the exercise and the holding of the Options and the sale of the Depositary Receipts derived from exercise of the Options, will be for the account of the Participant.

*Article 7.b.*

If the Option is not exercised, any tax and/or social security premiums paid will not be refunded or compensated for.

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**Adjustments**



#### Article 8

In case of any adjustment made by the Company with respect to the Options and/or the Plan the Company shall notify the Participant of the adjustments and its consequences to the Options in writing at least 10 days in advance.

#### Governing law

##### Article 9.a.

The Grant Letter shall be governed by the laws of the Netherlands.

##### Article 9.b.

All disputes arising in connection with the Grant Letter shall be exclusively submitted to the jurisdiction of the competent court in Amsterdam, the Netherlands.

#### Acceptance

##### Article 10

The Participant hereby declares to accept the Options granted to him including the conditions stipulated in the Grant Letter. This Grant Letter can be used as a Power of Attorney in accordance with article 7.2 of the Plan.

Duly signed in [·], on [·].

uniQure B.V.  
Name:

Participant  
Name

#### SCHEDULE 2: NOTICE OF EXERCISE (“NOTICE”)

To: uniQure B.V.

Exercise statement in accordance with Article 4.4 of the Plan.

1. Pursuant to the Grant on 5 April 2012 to the Optionholder,
2. the Participant hereby exercises Options.
3. The Option Exercise Price is € 0.61 [amount payable per Depositary Receipt]
4. and the Optionholder hereby agrees to pay an aggregate amount of € , on receipt of which the Company undertakes to issue or transfer the resulting number of Depositary Receipts, subject to the terms and conditions of the Plan.

Definitions used in the uniQure 2012 Stock Option Plan shall apply in this Notice.

Signed:  
Name:  
Date:

uniQure

**GRANT LETTER**

This Grant Letter is made on the [·] day of [··], [····].

**BY AND BETWEEN:**

1. [·], residing at [·], hereinafter referred to as “the Participant”;

and

2. uniQure B.V. (uniQure), a private limited liability company incorporated in the Netherlands having its official seat in Amsterdam, the Netherlands, hereinafter referred to as “the Company”;

Together hereinafter referred to as “**Parties**”; **WHEREAS:**

- The Company has implemented the uniQure 2012 Stock Option Plan (hereinafter referred to as “the Plan”).
- The Company hereby wishes to offer the Participant certain Options as set out herein, pursuant to Article 2 of the Plan, under the terms and conditions of the Plan.

**THE PARTIES HERETO AGREE AS FOLLOWS:****Definitions and interpretation***Article 1*

Capitalised terms used and not otherwise defined in this Grant Letter shall have the meanings given thereto in the Plan. The terms and conditions of the Plan are applicable to this Grant Letter, and are deemed to be incorporated in this Grant Letter by reference. By signing this Grant Letter, the Participant accepts the terms and conditions of the Plan. A copy of the Plan is attached hereto.

Where the provisions of this Grant Letter deviate from or are in conflict with the provisions and/or purpose of the Plan, the provisions of the Plan shall prevail, save in respect of references to “Eligible Employee” and or “employee” which shall be deemed to include individuals holding office as STAK Board members, and references in Article 8 of the Plan shall be interpreted with respect to holding such office.

**Grant of Options***Article 2*

Subject to the terms and conditions of the Plan and this Grant Letter, the Company hereby grants to the Participant [ · ] Options.

**Option Period***Article 3*

The Date of Grant of the Options is [·].

**Option Exercise Price***Article 4*

The Option Exercise Price is hereby established at [·] per Depositary Receipt, in accordance with section 2.3 of the Plan.

**Exercise***Article 5*

The Options can only be exercised if and to the extent the Options have vested, and are not expired or lapsed, all in accordance with Article 4 of the Plan, and subject to the other terms and conditions of the Plan.

**Non transferability***Article 6*

The Options granted to the Participant under this Grant Letter can not be transferred, pledged or encumbered in any way, either in full or in part. Breach of this article will cause the Option to lapse forthwith.

**Taxes, social security premiums***Article 7.a.*

Any wage or personal or corporate income tax, or social security premiums due in connection with the Options, including but not limited to any wage tax, income tax or social security premiums due in connection with the grant, the exercise and the holding of the Options and the sale of the Depositary Receipts derived from exercise of the Options, will be for the account of the Participant.

Article 7.b.

If the Option is not exercised, any tax and/or social security premiums paid will not be refunded or compensated for.

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**Adjustments**

Article 8

In case of any adjustment made by the Company with respect to the Options and/or the Plan the Company shall notify the Participant of the adjustments and its consequences to the Options in writing at least 10 days in advance.

**Governing law**

Article 9.a.

The Grant Letter shall be governed by the laws of the Netherlands.

Article 9.b.

All disputes arising in connection with the Grant Letter shall be exclusively submitted to the jurisdiction of the competent court in Amsterdam, the Netherlands.

**Acceptance**

Article 10

The Participant hereby declares to accept the Options granted to him including the conditions stipulated in the Grant Letter. This Grant Letter can be used as a Power of Attorney in accordance with article 7.2 of the Plan.

Duly signed in [·], on [·].

uniQure B.V. Name:	Participant Name
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113 HARTWELL AVENUE  
LEXINGTON, MASSACHUSETTS

LEASE SUMMARY SHEET

<b><u>Execution Date:</u></b>	July 24, 2013
<b><u>Tenant:</u></b>	uniQure, Inc., a Delaware corporation
<b><u>Landlord:</u></b>	King 113 Hartwell LLC, a Massachusetts limited liability company
<b><u>Building:</u></b>	The one story building known and numbered as 113 Hartwell Avenue, Lexington, Massachusetts. The Building consists of approximately 103,800 rentable square feet. The land on which the Building is located (the “ <b><u>Land</u></b> ”) is more particularly described in Exhibit 2 attached hereto and made a part hereof (such land, together with the Building, are hereinafter collectively referred to as the “ <b><u>Property</u></b> ”).
<b><u>Premises:</u></b>	Approximately 53,343 rentable square feet of space in the Building, as more particularly shown as hatched, highlighted or outlined on the plan attached hereto as <b><u>Exhibit 1A</u></b> and made a part hereof (the “ <b><u>Lease Plan</u></b> ”). Landlord and Tenant acknowledge and agree that the Premises contains two of the Building’s five loading bays, which two loading bays are dedicated to Tenant’s exclusive use, but such loading bays are not separately demised.
<b><u>Term Commencement Date:</u></b>	The date on which Landlord delivers the Premises to Tenant with Phase I of Landlord’s Work substantially complete, as defined in Section 3.2(c). Targeted for the date which is seventy-five (75) business days after the Execution Date (such 75 <sup>th</sup> business day, the “ <b><u>Target Date</u></b> ”).
<b><u>Rent Commencement Date:</u></b>	Seven (7) months after the Term Commencement Date.
<b><u>Expiration Date:</u></b>	The last day of the tenth (10 <sup>th</sup> ) Rent Year.
<b><u>Extension Terms:</u></b>	Subject to Section 1.2 below, two (2) extension terms of five (5) years each
<b><u>Landlord’s Contribution:</u></b>	Subject to Section 3.4 below, Seven Million Two Hundred Six Thousand Six Hundred Thirty-Nine and 30/100 Dollars (\$7,206,639.30)
<b><u>Permitted Uses:</u></b>	Subject to Section 3.3(f) below, general office, research, development, light manufacturing and laboratory use, and, subject to Legal Requirements, such other ancillary uses related to the foregoing.

<b><u>Base Rent:</u></b>	<b><u>RENT YEAR(1)</u></b>	<b><u>ANNUAL BASE RENT</u></b>	<b><u>MONTHLY PAYMENT</u></b>
	1	\$ 1,653,633.00	\$ 137,802.75
	2	\$ 1,706,976.00	\$ 142,248.00
	3	\$ 1,760,319.00	\$ 146,693.25
	4	\$ 1,813,662.00	\$ 151,138.50
	5	\$ 1,867,005.00	\$ 155,583.75
	6	\$ 1,920,348.00	\$ 160,029.00
	7	\$ 1,973,691.00	\$ 164,474.25
	8	\$ 2,027,034.00	\$ 168,919.50
	9	\$ 2,080,377.00	\$ 173,364.75
	10	\$ 2,133,720.00	\$ 177,810.00

(1) For the purposes of this Lease, the first “**Rent Year**” shall be defined as the period commencing as of the Rent Commencement Date and ending on the last day of the month in which the first (1<sup>st</sup>) anniversary of the Rent Commencement Date occurs; provided, however, that if the Rent Commencement Date occurs on the first day of a calendar month, then the first Rent Year shall expire on the day immediately preceding the first (1<sup>st</sup>) anniversary of the Rent Commencement Date. Thereafter, “Rent Year” shall be defined as any subsequent twelve (12) month period during the term of this Lease.

<b><u>Operating Costs and Taxes:</u></b>	See Sections 5.2 and 5.3
<b><u>Tenant’s Share:</u></b>	A fraction, the numerator of which is the number of rentable square feet in the Premises and the denominator of which is the number of rentable square feet in the Building. As of the Execution Date, Tenant’s Share is 51.39%, which shall only be subject to increase pursuant to Section 15 or an expansion of the Premises.
<b><u>Security Deposit/ Letter of Credit:</u></b>	\$1,240,224.75
<b><u>Guarantor:</u></b>	uniQure BV

EXHIBIT 1A	LEASE PLAN
EXHIBIT 1B	PLAN OF TENANT’S EXCLUSIVE PARKING SPACES AND COMMON PARKING AREA
EXHIBIT 1C	PLAN OF LOCATION OF TENANT’S EMERGENCY BACK-UP EQUIPMENT
EXHIBIT 1D	PLAN OF ROOFTOP PREMISES

EXHIBIT 1E	PLAN OF OUTDOOR PATIO LOCATION
EXHIBIT 1F	PLAN OF ROSO SPACE
EXHIBIT 1G	PLAN OF UNLEASED SPACE
EXHIBIT 2	LEGAL DESCRIPTION
EXHIBIT 3A	MATRIX
EXHIBIT 3B	LANDLORD WORK PLANS
EXHIBIT 3C	TENANT’S PROGRAM
EXHIBIT 3D	TENANT’S ARCHITECT, HVAC AND MEP ENGINEERS AND GENERAL CONTRACTOR
EXHIBIT 3E	LANDLORD’S WORK CONSTRUCTION SCHEDULE
EXHIBIT 4	FORM OF GUARANTY
EXHIBIT 5	FORM OF LETTER OF CREDIT
EXHIBIT 6	LANDLORD’S SERVICES
EXHIBIT 7	TENANT’S HAZARDOUS MATERIALS
EXHIBIT 8	RULES AND REGULATIONS
EXHIBIT 9	FORM OF NOTICE OF LEASE
EXHIBIT 10	PTDM
EXHIBIT 11	SIGNAGE GUIDELINES
EXHIBIT 12	MATTERS OF RECORD
EXHIBIT 13	FORM OF SNDA

THIS INDENTURE OF LEASE (this “**Lease**”) is hereby made and entered into on the Execution Date by and between Landlord and Tenant.

Each reference in this Lease to any of the terms and titles contained in any Exhibit attached to this Lease shall be deemed and construed to incorporate the data stated under that term or title in such Exhibit. All capitalized terms not otherwise defined herein shall have the meanings ascribed to them as set forth in the Lease Summary Sheet which is attached hereto and incorporated herein by reference.

## 1. LEASE GRANT; TERM; APPURTENANT RIGHTS; EXCLUSIONS

**1.1 Lease Grant.** Landlord hereby leases to Tenant, and Tenant hereby leases from Landlord, the Premises upon and subject to terms and conditions of this Lease, for a term of years commencing on the Term Commencement Date and, unless earlier terminated or extended pursuant to the terms hereof, ending on the Expiration Date (the “**Initial Term**”; the Initial Term and any duly exercised Extension Terms are hereinafter collectively referred to as the “**Term**”). Landlord represents and warrants that, except as listed on Exhibit 12 attached, there are no liens, encumbrances or other documents of record affecting the Property as of the Execution Date. Landlord represents and warrants that, as of the Execution Date, general office, laboratory, research and development and light manufacturing are permitted uses at the Property under all applicable zoning and land use laws, codes and ordinances (without regard to any Alterations, including without limitation Tenant’s Work).

### 1.2 Extension Terms.

(a) Provided (i) that subleases for more than fifty percent (50%) of the Premises are not then in effect (excluding any Permitted Transfers) (the “**Occupancy Condition**”); (ii) Tenant has not been in default of its obligations for an aggregate of thirty (30) days or more prior to the date of the Extension Notice, and (iii) no Event of Default is then continuing (1) as of the date of the Extension Notice (hereinafter defined), and (2) at the commencement of the applicable Extension Term (hereinafter defined), Tenant shall have the option to extend the Term for two (2) additional terms of five (5) years each (each, an “**Extension Term**”), commencing as of the expiration of the Initial Term, or the prior Extension Term, as the case may be. Tenant must exercise such option to extend by giving Landlord written notice (the “**Extension Notice**”) on or before the date (the “**Extension Deadline**”) that is twelve (12) months prior to the expiration of the then-current term of this Lease, *time being of the essence*. Upon the timely giving of such notice, the Term shall be deemed extended upon all of the terms and conditions of this Lease, except that Base Rent during each Extension Term shall be calculated in accordance with this Section 1.2, Landlord shall have no obligation to construct or renovate the Premises pursuant to Section 3, and Tenant shall have one (1) fewer option to extend the Term. If Tenant fails to give timely notice, as aforesaid, Tenant shall have no further right to extend the Term. Notwithstanding the fact that Tenant’s proper and timely exercise of such option to extend the Term shall be self-executing, the parties shall promptly execute a lease amendment reflecting such Extension Term after Tenant exercises such option. The execution of such lease amendment shall not be deemed to waive any of the conditions to Tenant’s exercise of its rights under this Section 1.2.

(b) The Base Rent during each Extension Term (the “**Extension Term Base Rent**”) shall be determined in accordance with the process described hereafter. Extension Term Base Rent shall be the fair market rental value of the Premises then demised to Tenant as of the commencement of the applicable Extension Term as determined in accordance with the process described below, for renewals of combination laboratory and office space in the Town of Lexington, Massachusetts, of equivalent quality, size, utility and location, with the length of the Extension Term, the credit standing of Tenant and all other

relevant factors to be taken into account, provided, however, any improvements paid for by Tenant shall not be taken into account. Within thirty (30) days after receipt of the Extension Notice or in any event within 30 days following any request from Tenant, Landlord shall deliver to Tenant written notice of its reasonable, good faith determination of the Extension Term Base Rent for the applicable Extension

Term ("**Landlord's Rent Estimate**"). Tenant shall, within thirty (30) days after receipt of such notice (or, if received prior the Extension Deadline, no later than the timely giving of its Extension Notice), notify Landlord in writing whether Tenant accepts or rejects Landlord's determination of the Extension Term Base Rent ("**Tenant's Response Notice**"). If Tenant fails timely to deliver Tenant's Response Notice and has timely given its Extension Notice, Landlord's determination of the Extension Term Base Rent shall be binding on Tenant.

(c) If and only if Tenant's Response Notice and Extension Notice is timely delivered to Landlord and indicates both that Tenant rejects Landlord's determination of the Extension Term Base Rent and desires to submit the matter to arbitration, then the Extension Term Base Rent shall be determined in accordance with the procedure set forth in this Section 1.2(c). In such event, within ten (10) days after receipt by Landlord of Tenant's Response Notice indicating Tenant's desire to submit the determination of the Extension Term Base Rent to arbitration, Tenant and Landlord shall each notify the other, in writing, of their respective selections of a commercial real estate broker or appraiser (respectively, "**Landlord's Appraiser**" and "**Tenant's Appraiser**"). Landlord's Appraiser and Tenant's Appraiser shall then jointly select a third appraiser (the "**Third Appraiser**") within ten (10) days of their appointment. All of the brokers/appraisers selected shall be individuals with at least ten (10) consecutive years' commercial experience in the area in which the Premises are located, and in the case of appraisers, shall be members of the Appraisal Institute (M.A.I.). The Third Appraiser shall not have acted in any capacity for either Landlord or Tenant within ten (10) years of his or her selection. The three appraisers shall determine the Extension Term Base Rent in accordance with the requirements and criteria set forth in Section 1.2(b) above, employing the method commonly known as Baseball Arbitration, whereby Landlord's Appraiser and Tenant's Appraiser each sets forth its determination of the Extension Term Base Rent as defined above, and the Third Appraiser must select one or the other (it being understood that the Third Appraiser shall be expressly prohibited from selecting a compromise figure). Landlord's Appraiser and Tenant's Appraiser shall deliver their determinations of the Extension Term Base Rent to the Third Appraiser within five (5) days of the appointment of the Third Appraiser and the Third Appraiser shall render his or her decision within ten (10) days after receipt of both of the other two determinations of the Extension Term Base Rent. The Third Appraiser's decision shall be binding on both Landlord and Tenant. Each party shall bear the cost of its own appraiser and the cost of the Third Appraiser shall be paid by the party whose determination is not selected.

### 1.3 Appurtenant Rights.

(a) **Common Areas.** Subject to the terms of this Lease and the Rules and Regulations (hereinafter defined), Tenant shall have, as appurtenant to the Premises, rights to use in common with others entitled thereto, the following areas (such areas are hereinafter referred to as the "**Common Areas**"): (i) the common hallway of the Building serving the Premises (the "**Common Hallway**"), (ii) common walkways and driveways necessary for access to the Building, (iii) risers, shafts, chases, and conduits designated by Landlord for use by tenants and/or other occupants, (iv) parking areas as further described in the immediately following paragraph, (v) areas of the Building and on the rooftop of the Building necessary for access to the Rooftop Premises and/or the Common Hallway, and (vi) other areas and facilities designated by Landlord from time to time for the common use of tenants of the Building, if any; and except as provided under common law or as expressly set forth in this Lease, no other appurtenant rights or easements.

(b) **Parking.** During the Term, Landlord shall, subject to the terms hereof, make available up to one hundred sixty-five (165) parking spaces for Tenant's use in the parking areas serving the Building at no charge; provided that the foregoing shall not be deemed to exclude costs associated with the parking areas from Operating Costs or Taxes to the extent not otherwise excluded under Section 5.2(b). The number of parking spaces in the parking areas reserved for Tenant, as modified pursuant to this Lease or as otherwise permitted by Landlord, are hereinafter referred to as the "**Parking Spaces**." Tenant shall have no right to hypothecate or encumber the Parking Spaces, and shall not sublet, assign, or

otherwise transfer the Parking Spaces other than to employees of Tenant occupying the Premises or a transferee pursuant to an approved Transfer (or Transfer not requiring approval) under Section 13 of this Lease. Approximately twenty-nine (29) of the Parking Spaces shall be reserved near the main entrance to the Premises as shown on Exhibit 1B for Tenant's exclusive use, Tenant hereby acknowledging that some of such 29-spaces are designated as handicapped parking and/or carpool parking as shown on Exhibit 1B. Tenant may, subject to Landlord's reasonable approval, clearly label such 29 spaces as reserved for Tenant by Landlord. The remainder of the Parking Spaces shall be located in the area designated as "Common Parking" on Exhibit 1B, subject to the provisions of this Section 1.3(b). Subject to the foregoing, and subject to Landlord's right to reserve parking for other tenants of the Building in the areas shown on Exhibit 1B as "Reserved Available Parking" and "Quanterix Parking," (as such reserved parking for other tenants of the Building may be relocated to the Common Parking area (or elsewhere) by Landlord in connection with any Future Development), said Parking Spaces will be on an unassigned, non-reserved basis. All of the Parking Spaces shall be subject to such reasonable Rules and Regulations as may be in effect for the use of the parking areas from time to time. Notwithstanding anything to the contrary contained herein, (i) Landlord shall have the right, during the performance of Landlord's restoration obligations set forth in Section 15 below, to temporarily relocate all or any portion of the Parking Spaces to the parking areas located at 101 Hartwell Avenue, 4 Hartwell Place and/or 91 Hartwell Avenue, and (ii) Landlord shall have the one-time right, upon at least six (6) months' written notice to Tenant, in connection with Future Development (as defined in Section 2.2), to temporarily relocate all or any portion of the Parking Spaces located in the Common Parking area to the parking areas located at 101 Hartwell Avenue, 4 Hartwell Place and/or 91 Hartwell Avenue for a period of no more than two (2) years. Landlord shall use reasonable efforts to stage any Future Development in a manner that minimizes the duration of such temporary relocation.

(c) **Rooftop Premises.** During the Term, Tenant shall have the right to use the portion of the rooftop of the Building shown on the plan attached hereto as Exhibit 1D (the "**Rooftop Premises**") for the installation and operation of mechanical and communications equipment (A) serving only the Premises, (B) approved by Landlord and (C) purchased and installed by Tenant in accordance with the terms of this Lease (any equipment installed within the Rooftop Premises, as the same may be modified, altered or replaced during the Term, is collectively referred to herein as "**Tenant's Rooftop Equipment**"). Landlord's approval of such equipment shall not be unreasonably withheld, conditioned or delayed. Landlord acknowledges that the equipment shown on Exhibit 1D attached hereto has been approved by Landlord (subject to the provisions of Sections 3 (if applicable) and 11 governing the installation thereof. Tenant shall operate Tenant's Rooftop Equipment in a manner that does not interfere with (x) any make-up air installations to the extent installed prior to the date of installation of Tenant's Rooftop Equipment, (y) any other tenant's rooftop equipment to the extent installed prior to the date of installation of Tenant's Rooftop Equipment or (z) base building equipment operated by Landlord to the extent installed prior to the date of installation of Tenant's Rooftop Equipment. Any installation of Tenant's Rooftop Equipment (i) will not affect the structural integrity of the Building or impact the roof or the roof membrane in any manner; (ii) shall be adequately screened so as to minimize the visibility of such equipment; and (iii) shall be adequately sound-proofed to meet all requirements of Legal Requirements. Tenant shall not install or operate Tenant's Rooftop Equipment until Tenant has obtained and submitted to Landlord copies of all required

governmental permits, licenses, and authorizations necessary for the installation and operation thereof. In addition, Tenant shall comply with all reasonable construction Rules and Regulations promulgated by Landlord in connection with the installation, maintenance and operation of Tenant's Rooftop Equipment. Landlord shall have no obligation to provide any services including, without limitation, electric current or gas service, to the Rooftop Premises or to Tenant's Rooftop Equipment, it being understood and agreed that Tenant shall be responsible for constructing any risers, shafts, chases, or conduits necessary to connect the necessary services from the Premises to Tenant's Rooftop Equipment. Tenant shall be responsible for repairing and maintaining Tenant's Rooftop Equipment at Tenant's sole cost and, subject to the provisions of Section 14.5, the cost of repairing any damage to the Building, or the cost of any necessary improvements to the Building, caused by or as a result of the installation, replacement and/or removal of Tenant's Rooftop Equipment.

Landlord makes no warranties or representations to Tenant as to the suitability of the Rooftop Premises for the installation and operation of Tenant's Rooftop Equipment. In the event that at any time during the Term, the operation and/or periodic testing of any other rooftop equipment installed after Tenant's Rooftop Equipment interferes with Tenant's make-up air installations or the operation of the Tenant's Rooftop Equipment or the business operations of Tenant, then Landlord shall, upon notice from Tenant, cause (if Building rooftop equipment) or use commercially reasonable efforts to cause the operator of the interfering equipment (if other tenant's rooftop equipment) to cease such testing or interference, as applicable. In the event that at any time during the Term, Landlord determines, in its bona fide and good faith business judgment, that the operation and/or periodic testing of Tenant's Rooftop Equipment unreasonably interferes with the operation of the Building or the business operations of any of the occupants of the Building (other than interference with rooftop equipment installed after the installation of Tenant's Rooftop Equipment), recognizing the use of the Building for the Permitted Uses, then Tenant shall, upon notice from Landlord, cease using such interfering equipment and cause all further testing of Tenant's Rooftop Equipment to occur after normal business hours (hereinafter defined). From and after the Execution Date, Landlord shall include substantially similar provisions governing rooftop equipment in any other leases at the Building (giving priority to previously installed rooftop equipment vis-à-vis subsequently installed rooftop equipment) and shall not enforce such provisions in a discriminatory manner.

(d) **On-Site Generator.** Subject to Legal Requirements and Landlord's prior written approval of plans and specifications therefor, Tenant may install, operate and maintain, in the location shown on the plan attached hereto as Exhibit 1C or another location reasonably approved by Landlord (the "**Generator Location**"), two 150KW emergency generators or, at Tenant's election, one 350 KW generator (the "**Emergency Back-up Equipment**") at Tenant's sole cost and expense; provided, however, that if any Emergency Back-up Equipment is located in the parking areas, any parking spaces used /lost as a result thereof shall be included in Tenant's parking allotment as described in Section 1.3(b) above. Landlord shall have no obligation to provide any services including, without limitation, electric current or gas service, to the Emergency Back-up Equipment, provided, however, subject to Legal Requirements and Landlord's prior written approval of plans and specifications therefor, Tenant may also install, maintain and operate necessary utility connections between the Emergency Back-up Equipment and the Premises (which utility connections shall be deemed part of the Emergency Back-up Equipment). Landlord may, in its sole and absolute discretion, require Tenant, at Landlord's cost, to relocate any or all of the Emergency Back-up Equipment to a location with comparable functionality, which relocation shall be performed by Tenant within a reasonable period following such request (taking into account any reasonable time necessary to obtain permits and approvals for such work, Tenant hereby agreeing to use diligent good faith efforts to obtain the same and to promptly commence and prosecute to completion such relocation thereafter. Landlord agrees to require such relocation no more than once during the Term (provided that such limitation shall not apply to temporary relocations required in connection with any required maintenance, repair or replacement by Landlord.) Landlord's approval of the Emergency Back-up Equipment shall not be unreasonably withheld, conditioned or delayed. Tenant shall be responsible for the cost of repairing and maintaining the Emergency Back-up Equipment in good order, condition and repair and in compliance with Legal Requirements and, subject to the provisions of Section 14.5, for the cost of repairing any damage to the Property, or the cost of any necessary improvements to the Property, caused by or as a result of the installation, replacement and/or removal of the Emergency Back-up Equipment. Landlord makes no warranties or representations to Tenant as to the suitability of the Generator Location for the installation and operation of the Emergency Back-up Equipment. Tenant shall not install or operate the Emergency Back-up Equipment until Tenant has obtained and submitted to Landlord copies of all required governmental permits, licenses, and authorizations necessary for the installation and operation thereof. In addition, Tenant shall comply with all reasonable Rules and Regulations in connection with the installation, maintenance and operation of the Emergency Back-up Equipment.

(e) **Outdoor Patio.** Subject to the Rules and Regulations and the provisions of this

Lease, Landlord agrees that Tenant shall have, as appurtenant to the Premises, the exclusive right to use the area adjacent to the Premises designated by Landlord, as more particularly shown on the plan attached hereto as "Outdoor Patio Location" on Exhibit 1E attached hereto (the "**Outside Seating Area**") throughout the Term hereof. Notwithstanding anything to the contrary contained herein, Landlord shall have the right, upon as much notice as is practicable under the circumstances and in any event no less than forty-eight (48) hours (except that no notice shall be required in the event of an emergency) to close all or any portion of the Patio Area in connection with the performance of repairs, maintenance, and/or construction (if such closure cannot be reasonably avoided in connection with such repairs, maintenance and/or construction). Tenant shall install, at its sole cost and expense, all desired furniture, equipment and lighting (collectively, "**Furniture**") in the Outside Seating Area. Tenant acknowledges and agrees that (A) the Furniture shall meet the standards of quality and appearance consistent with the first-class nature of the Building; and (B) Tenant shall be solely responsible for any destruction, damage, theft or vandalism of, or to, the Furniture. Tenant hereby covenants and agrees that it shall not: (A) restrict access to the Building or pedestrian flow through the Common Areas outside the Outside Seating Area; (B) erect or place any canopy or other enclosure or covering on the Outside Seating Area; (C) permit any music or other similar sounds to be heard in the Outside Seating Area without Landlord's prior written approval, which shall not be unreasonably withheld, conditioned or delayed; or (D) permit loitering in the Outside Seating Area by persons who are not invitees of Tenant. Tenant shall clean and maintain in good order, condition and repair the Outside Seating Area and Furniture and shall remove all Trash (hereinafter defined) generated from the Outside Seating Area on a daily basis or more frequently as needed. Immediately upon Landlord's demand therefor, Tenant shall reimburse Landlord for the cost of repairs or restoration of the Common Areas arising out of Tenant's use of the Outside Seating Area. To the extent applicable, all provisions of this Lease shall apply to Tenant's use and occupancy of the Outside Seating Area.

#### **1.4 Tenant's Access.**

(a) From and after the Term Commencement Date and until the end of the Term, Tenant shall have access to the Premises twenty-four (24) hours a day, seven (7) days a week, subject to Legal Requirements, the Rules and Regulations, the terms of this Lease and matters of record as of the Execution Date.

(b) Upon at least two (2) business days' notice, Tenant shall have the right to access the Premises with a Landlord representative present, at Tenant's sole risk, after the Execution Date but prior to the Term Commencement Date for taking measurements and other non-construction activities to facilitate space planning, provided such access does not materially interfere with the preparation for or performance of Landlord's Work (hereinafter defined). Tenant shall, prior to the first entry to the Premises pursuant to this Section 1.4(b), provide Landlord with certificates of insurance evidencing that the insurance required in

Section 14 hereof is in full force and effect and covering any person or entity entering the Building. Tenant shall defend, indemnify and hold the Landlord Parties (hereinafter defined) harmless from and against any and all Claims (hereinafter defined) for injury to persons or property to the extent resulting from or relating to Tenant's access to the Premises prior to the Term Commencement Date as provided under this Section 1.4(b). Tenant shall coordinate any access to the Premises prior to the Term Commencement Date with Landlord's property manager.

**1.5 Notice of Lease.** Neither party shall record this Lease, but each of the parties hereto agrees to join in the execution, in recordable form, of a statutory notice of lease in substantially the form attached hereto as Exhibit 9, which notice of lease may be recorded by Tenant with the Middlesex South Registry of Deeds and/or filed with the Registry District of the Land Court, as appropriate (collectively, the "**Registry**," at Tenant's sole cost and expense (provided, however, that Landlord shall provide such evidence of authority as is reasonably required for the filing of the notice of lease at Landlord's sole cost and expense). If a notice of lease was previously recorded with the Registry, upon the expiration or earlier termination of this Lease, Landlord shall deliver to Tenant a notice of termination

of lease and Tenant shall promptly execute and deliver the same to Landlord for Landlord's execution and recordation with the Registry, which obligation shall survive the expiration or earlier termination of the Lease.

**1.6 Exclusions.** The following are expressly excluded from the Premises and reserved to Landlord: all the perimeter walls of the Premises (except the inner surfaces thereof), the Common Areas, and any space in or adjacent to the Premises used for shafts, stacks, pipes, conduits, wires and appurtenant fixtures, fan rooms, ducts, electric or other utilities, sinks and other facilities serving portions of the Building other than the Premises, and the use of all of the foregoing, except as expressly permitted pursuant to Section 1.3(a) above.

## **2. RIGHTS RESERVED TO LANDLORD**

**2.1 Additions and Alterations.** Landlord reserves the right, at any time and from time to time, to make such changes, alterations, additions, improvements, repairs or replacements in or to the Property (including the Premises but, with respect to the Premises, only for purposes of repairs, maintenance, replacements and other rights expressly reserved to Landlord hereunder) and the fixtures and equipment therein, as well as in or to the street entrances and/or the Common Areas, as it may deem necessary or desirable, provided, however, that there be no material obstruction of access to, or interference (other than de minimis interference consistent with the undertaking of ordinary alterations, repairs and maintenance in a first class office and laboratory buildings) with the use and enjoyment of, the Premises and Tenant's express appurtenant rights in the Property described herein (subject to the provisions of Section 1.3, above) and such changes are consistent with an occupied first-class office and laboratory building in the area. Subject to the foregoing, and subject to Section 1.3(b) above, Landlord expressly reserves the right to temporarily close all, or any portion, of the Common Areas for the purpose of making repairs or changes thereto, provided that the duration of such closure will be limited to a reasonable period of time in light of the nature of the repairs or changes being made thereto, consistent with an occupied first-class office and laboratory building. In no event shall Landlord exercise any of its rights under this Section 2.1 without giving Tenant reasonable prior notice of the same. In no event shall Landlord be permitted pursuant to this Section 2.1 or otherwise to construct additional leasable areas above the Premises.

**2.2 Additions to the Property.** Landlord may at any time or from time to time construct additional buildings and related site improvements (collectively, the "**Future Development**") in the portion of the Property comprised of the Common Parking areas shown on Exhibit 1B and the undeveloped land to the west-north-west of the Building, provided that Future Development shall not require use of the Premises or any portion of the Building connected to or serving the Premises. In addition, but subject to Section 1.3 above, Landlord may change the location or arrangement of any improvement outside the Building in or on the Property or all or any part of the Common Areas, or add or deduct any land to or from the Property. There shall be no increase in Tenant's obligations or interference with Tenant's rights under this Lease in connection with the exercise of the foregoing reserved rights (other than de minimis impacts not inconsistent with occupied first class office and laboratory buildings).

In connection with any Future Development, in no event shall Tenant be denied reasonable access to the parking required under Section 1.3(b), as such parking may be relocated pursuant thereto. Landlord agrees, in connection with any such development or redevelopment, to utilize all commercially reasonable efforts to mitigate the impacts of earthwork and other construction activities on Tenant's business operations consistent with standards for occupied first-class suburban office, laboratory and research and development projects. Landlord shall prepare and deliver a reasonable construction mitigation plan after consulting with Tenant, which plan shall incorporate such measures as are reasonably required to maintain and operate Tenant's business in the Premises in a manner consistent with first class office and laboratory use. Tenant shall have the right to approve such mitigation plan, such approval not to be

unreasonably withheld, conditioned or delayed (and any objections to such plan must be specifically described in writing so that Landlord may respond to them). Landlord will cause all blasting, pile driving or other work resulting in vibrations within the Premises in excess of customary levels of activity in an occupied first class office and laboratory building to take place during normal business hours unless required by Legal Requirements (including without limitation the Town of Lexington), Landlord hereby agreeing not to request that the Town or any other governmental authority require that such blasting occur at any particular time) and such work must be scheduled with at least 30 days' prior written notice to Tenant.

Landlord and Tenant each hereby acknowledges and agrees that, in connection with Future Development, (a) Landlord shall have the right to subject the Land and the improvements located now or in the future located thereon to a commercial condominium regime ("**Condominium**") on terms and conditions consistent with first class office and laboratory parks in the Route 128 area, in which the Building shall be a single unit; (b) upon Landlord's request in connection with the recording of the Master Deed for the Condominium and the Unit Deed for the Building, Tenant shall execute a reasonable instrument in recordable form making this Lease subject and subordinate to the Master Deed and other documents evidencing the Condominium (collectively, the "**Condo Documents**") provided that such Condo Documents continue to provide Tenant with all of the rights and obligations contained in this Lease (e.g. the appurtenant right to use all Common Areas) and the Condo Documents comply with the provisions of this Section 2.2; (c) Landlord shall have the right to enter into, and subject the Property to the terms and conditions of, a reciprocal easement agreement with any one or more of the neighboring property owners in order to create a commercial campus-like setting ("**REA**") provided that such REA continues to provide Tenant with all of the rights and obligations contained in this Lease as of the Execution Date (e.g. the appurtenant right to use all Common Areas) and the REA complies with the provisions of this Section 2.2; (d) Landlord shall submit to Tenant for Tenant's approval drafts of the Condo Documents and the REA (and any amendments thereto) prior to their execution; (e) Tenant shall have the right to notify Landlord within twenty (20) days after receipt of the draft Condo Documents and/or REA (or any amendments thereto) of Tenant's disapproval thereof, but only to the extent such draft(s) (i) adversely affect Tenant's use of, or access to, the Premises, the Building systems or the Rooftop Premises in more than a de minimis manner, (ii) adversely affect in more than a de minimis manner the operation of Tenant's business from the Premises in accordance with the terms of this Lease, or



Tenant's rights under and pursuant to the terms of this Lease, including without limitation Tenant's rights with respect to the Common Areas, and/or (iii) result in any increase in Tenant's payment or other obligations under this Lease in more than a de minimis manner; (f) upon Landlord's request in connection with the recording of the REA, Tenant shall execute a commercially reasonable instrument in recordable form making this Lease subject and subordinate to the REA; (g) Landlord shall have the right to subdivide the Property so long as Tenant continues to have all of the rights and obligations contained in this Lease (e.g. the appurtenant right to use all Common Areas); and (h) Tenant shall execute such reasonable documents (which may be in recordable form) evidencing the foregoing promptly upon Landlord's request. Landlord shall reimburse Tenant for the reasonable out-of-pocket costs incurred by Tenant, if any, to review and comment on any documents or instruments presented to Tenant pursuant to this paragraph.

**2.3 Name and Address of Building.** Landlord reserves the right at any time and from time to time to change the name or address of the Building and/or the Property, provided Landlord gives Tenant at least three (3) months' prior written notice thereof. Notwithstanding the foregoing to the contrary, so long as Tenant's Share is at least 50%, Landlord shall not voluntarily change the address of the Building without Tenant's prior written consent, not to be unreasonably withheld, conditioned or delayed. Within thirty (30) days after receipt of a reasonably detailed invoice, Landlord shall reimburse Tenant for up to \$2,500 of the reasonable out-of-pocket costs incurred by Tenant in connection with any such change. Landlord shall not name the Building after any tenant or occupant and any such name shall be consistent with first class office and laboratory buildings in the area.

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**2.4 Landlord's Access.**

(a) Subject to the terms hereof, Tenant shall (i) upon as much advance notice as is practical under the circumstances, and in any event at least twenty-four (24) hours' prior written notice (except that no notice shall be required in emergency situations), permit Landlord and any holder of a Mortgage (hereinafter defined) (each such holder, a "**Mortgagee**"), and their agents, representatives, employees and contractors, to have reasonable access to the Premises at all reasonable hours for the purposes of inspection, making repairs, replacements or improvements in or to the Premises or the Building or equipment therein (including, without limitation, sanitary, electrical, heating, air conditioning or other systems), complying with all applicable laws, ordinances, rules, regulations, statutes, by-laws, court decisions and orders and requirements of all public authorities (collectively, "**Legal Requirements**"), or exercising any right reserved to Landlord under this Lease requiring such entry (but expressly excluding the right to store within the Premises any materials, tools and equipment); (ii) permit Landlord and its agents and employees, at reasonable times, upon reasonable advance notice, to show the Premises during normal business hours (i.e. Monday — Friday 8 A.M. - 6 P.M., excluding holidays) to any prospective Mortgagee or purchaser of the Building and/or the Property or of the interest of Landlord therein, and, during the last twelve (12) months of the Term, prospective tenants; (iii) upon reasonable prior written notice from Landlord, permit Landlord and its agents, at Landlord's sole cost and expense, to perform environmental audits, environmental site investigations and environmental site assessments ("**Site Assessments**") in, on, under and at the Premises and the Land, it being understood that Landlord shall repair any damage arising as a result of the Site Assessments, and such Site Assessments may include both above and below the ground testing and such other tests as may be necessary or appropriate to conduct the Site Assessments, Landlord hereby agreeing to provide Tenant with a copy of the resulting Site Assessment reports when issued in its final form, and (iv) to the extent that it is necessary to enter the Premises in order to access any area that serves any portion of the Building outside the Premises, then Tenant shall, upon as much advance notice as is practical under the circumstances, and in any event at least twenty-four (24) hours' prior written notice (except that no notice shall be required in emergency situations), permit contractors engaged by other occupants of the Building to pass through the Premises in order to access such areas but only if accompanied by a representative of Landlord.

(b) Except in emergency situations, anyone who has access to any portion of the Premises pursuant to this Section 2.4 after Tenant has first commenced to use the Premises for the Permitted Uses may, at Tenant's election, be subject to Tenant's reasonable security measures and protocols, which may include requiring that any party accessing the Premises under Section 2.4(a)(ii) and (iv) execute a commercially reasonable confidentiality agreement, requiring the wearing of an ID badge, and obligating visitors to comply with reasonable protocols so as to protect confidential information contained within the Premises. Except in the event of an emergency threatening personal injury or damage to property or a violation of any Legal Requirement, and except as otherwise approved by Tenant, any entry in the Premises must be done in the presence of a representative of Tenant so long as Tenant makes such representative available in a reasonable manner. Tenant may prohibit access pursuant to this Section 2.4 to certain areas of the Premises ("**Secure Areas**") reasonably identified by Tenant in a prior written notice to Landlord from time to time, which notice shall set forth the reasonable basis on which Tenant has determined that access must be prohibited to such areas in non-emergency situations. In no event shall Landlord be deemed to be in default hereunder, nor shall Landlord have any liability hereunder, to the extent that Landlord is prevented from performing any of its obligations as a result of its inability to access the Secure Areas in non-emergency situations. Notwithstanding the foregoing, in case of emergency, Landlord may enter any part of the Premises (including without limitation the Secure Areas) without prior notice or a Tenant's representative; provided that Landlord provides Tenant with notice of such entry as soon as reasonably possible thereafter and Landlord takes reasonable precautions to protect the health and safety of its entrants. Nothing in this paragraph will be construed as permitting Tenant to prohibit such access to any portion of the Premises other than Secure Areas.

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(c) Except in the event of an emergency, (i) Landlord shall schedule any of such access under this Section 2.4 with Tenant in advance; and (ii) to the extent such access shall, in Tenant's reasonable judgment, be likely to cause material interference with Tenant's business operations, Landlord shall, at Tenant's request, schedule any such entry pursuant to Sections 2.4(a)(i) and (iii) after normal business hours.

(d) Except to the extent arising as a result of the negligence or willful misconduct of the Tenant Parties, Landlord shall, subject to Section 14.5 below, defend, indemnify and hold Tenant harmless from and against any and all Claims (as defined below) resulting from or relating to access to the Premises as provided under this Section 2.4.

(e) Any provision of this Lease that requires or gives Landlord the right to enter the Premises during the Term shall be governed by the provisions of this Section 2.4 and this Section 2.

**2.5 Pipes, Ducts and Conduits.** Tenant shall permit Landlord to erect, use, maintain and relocate pipes, ducts and conduits in and through the Premises (other than those exclusively serving Tenant), provided the same are located to the exterior of any interior walls, above any drop ceilings or otherwise the lowest level of the roof structure, or below the floor, do not reduce the floor area or materially adversely affect the appearance thereof.

**2.6 Minimize Interference.** Except in the event of an emergency, Landlord shall use commercially reasonable efforts to minimize any interference with Tenant's business operations and use and occupancy of the Premises in connection with the exercise any of the foregoing rights under this Section 2.

**3. CONDITION OF PREMISES; CONSTRUCTION.**

**3.1 Condition of Premises.** Landlord shall deliver the Premises to Tenant (a) broom clean, (b) free of all personal property and occupants, (c) in compliance with Legal Requirements, (d) free of Hazardous Materials in excess of Reportable Concentrations and Reportable Quantities (as defined in Environmental Laws (hereinafter defined)), and (e) with the structural elements of the Building, the electric service, plumbing and life/safety systems, and other systems serving the Building in general, the Common Areas, the roof and roof system of the Building, the exterior wall system, and the exterior windows of the Premises weather tight and in good repair and working order. Subject to the foregoing, and subject to Landlord's obligation to perform Landlord's Work (hereinafter defined), Tenant acknowledges and agrees that Tenant is leasing the Premises in their "AS IS," "WHERE IS" physical condition and with all faults on the Execution Date, without representations or warranties, express or implied, in fact or by law, of any kind.

**3.2 Landlord's Work.**

(a) Performance. Subject to delays due to Force Majeure (hereinafter defined) and subject to any act or (where Tenant has a duty to act) omission by Tenant and/or Tenant's agents, servants, employees, consultants, contractors, subcontractors, licensees and/or subtenants (collectively with Tenant, the "**Tenant Parties**") which causes an actual delay in the performance of Landlord's Work (a "**Tenant Delay**") (provided that no Tenant Delay shall be deemed to occur until Landlord gives Tenant written notice of the event giving rise to such claimed Tenant Delay and a reasonable description of the same), Landlord, at Landlord's sole cost and expense, shall perform the work ("**Landlord's Work**") marked with an "x" in the Landlord column of the matrix attached hereto as Exhibit 3A (the "**Matrix**") and otherwise substantially in accordance with the complete, coordinated construction documents listed

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on Exhibit 3B (the "**Landlord Work Plans**"). The Landlord Work Plans may not be modified or changed in any material manner without Tenant's reasonable approval, provided, however, in no event shall any changes to the Landlord's Work Plans modify or be inconsistent with the requirements set forth on the Matrix. The weekly meetings described in Section 3.2(b) below shall include discussions of any changes to Landlord's Work (other than minor changes in the nature of field work). Landlord shall promptly provide Tenant with copies of any changes in the Landlord Work Plans. Following the approval by Landlord of Tenant's Schematic Plans, Landlord shall reimburse Tenant for any reasonable increase in the out-of-pocket costs of the design and construction of Tenant's Work to the extent resulting directly from any material changes in the Landlord Work Plans (it being understood that minor changes in the nature of field work shall not be deemed material) as reasonably documented by Tenant to Landlord so long as Tenant notified Landlord of the need therefor reasonably in advance of implementing the same, giving Landlord a reasonable opportunity to attempt to eliminate the need for such changes to Tenant's Plans.

(b) Phases; Performance. The portions of Landlord's Work not marked with an asterisk in the Matrix ("**Phase I of Landlord's Work**") shall be performed before the Term Commencement Date. The portions of Landlord's Work marked with an asterisk in the Matrix ("**Phase II of Landlord's Work**") may be performed after the Term Commencement Date. Landlord's Work shall be performed by B.W. Kennedy Company (or such other general contractor selected by Landlord) under a construction contract with Landlord. Landlord's Work will be designed and constructed in a good and workmanlike manner, and in full compliance with all Legal Requirements. Landlord will procure in a timely fashion and thereafter maintain all necessary approvals and permits from all state and local governmental agencies having authority over the construction of Landlord's Work. Landlord shall provide Tenant with copies of such permits (if already issued) or after the issuance thereof, as applicable. Landlord shall (A) commence Landlord's Work before or promptly after the Execution Date, and (B) use commercially reasonable diligent efforts to substantially complete Landlord's Work on or before the Target Date and in accordance with the construction schedule attached hereto as Exhibit 3E, provided that Landlord's failure in any way to adhere to such schedule shall not be deemed a default by Landlord, it being understood and agreed that Tenant's only remedies for failure to timely perform Landlord's Work are set forth in Section 3.2(e) below. Landlord shall schedule and conduct weekly meetings regarding the scheduling, progress, performance and construction of Landlord's Work. Periodically between such meetings, at Tenant's request, Landlord shall provide Tenant with status updates regarding the progress of Landlord's Work. During the course of design and construction, Landlord shall cause the construction schedule for Landlord's Work to be updated periodically to reflect the actual progress of design and construction, as applicable, and shall cause such updates to be delivered to Tenant at the next weekly meeting after Landlord's receipt thereof, but such updates shall not result in or be deemed to constitute Tenant's approval of any failure to timely substantially complete Landlord's Work in the absence of a Tenant Delay or Force Majeure.

(c) Substantial Completion; Punchlist Items. "**Substantially complete**," when referring to Landlord's Work, shall mean that Tenant has received a certificate of substantial completion from Landlord's architect stating that the applicable phase of Landlord's Work (i.e, Phase I of Landlord's Work or Phase II of Landlord's Work) is substantially complete, the parties acknowledging that there shall be a separate determination of substantial completion for each of such phases and a separate Punchlist for each of such phases. With respect to Phase I of Landlord's Work, "substantially complete" shall mean that Phase I of Landlord's Work is completed, other than work on the Punchlist applicable thereto, and that Tenant can promptly commence Tenant's Work and prosecute the same without unreasonable interference on account of the completion of the Punchlist Items for the Phase I of Landlord's Work. With respect to all of Landlord's Work, "substantially completed" shall mean that such work is completed, other than work on the Punchlists, and that no component of Landlord's Work (whether Phase I or Phase II) and whether on the Punchlist or not, prevents the issuance of a certificate of

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occupancy for the Premises. Promptly following substantial completion of each phase of Landlord's Work, Landlord shall provide Tenant with the applicable punchlist prepared by Landlord's architect (the "**Punchlist**") incorporating those items jointly identified by Landlord and Tenant during their joint inspection of Landlord's Work, of outstanding items which (a) need to be performed to complete such phase of Landlord's Work, (b) do not individually or in the aggregate prevent the prosecution of Tenant's Work (with respect to the Phase I of Landlord's Work), and do not individually or in the aggregate adversely affect the use and occupancy of the Premises for the normal conduct of the Permitted Uses or Tenant's rights hereunder with respect to the Common Areas (with respect to the entire Landlord's Work), all in a manner consistent with Punchlist items typically found in a first class office and laboratory facility (the "**Punchlist Items**"). Promptly after substantial completion of each phase of Landlord's Work, Landlord and Tenant shall jointly inspect the Premises. Subject to Force Majeure and Tenant Delays, Landlord shall complete all Punchlist Items within thirty (30) days of the date of the applicable Punchlist (other than (A) seasonal items, such as landscaping, requiring a longer period, and (B) Punchlist Items relating to Phase II of Landlord's Work which do not relate to the parking areas or other areas visible from the Premises, in each case, which shall be completed as soon as reasonably practicable), provided that Tenant reasonably cooperates (without requiring Tenant to incur any costs or to alter its operations in the Premises in more than a de minimis manner except to the extent necessary for Tenant to perform its obligations under Section 11.3 below) in connection with the completion of such Punchlist Items. Prior to substantial completion of each phase of Landlord's Work, Landlord shall obtain all applicable municipal sign-offs and/or approvals of such phase of Landlord's Work, if any (except to the extent that any such sign-off and/or approval is unavailable due to the status of Tenant's Work, in which event Landlord shall obtain the same as soon as possible thereafter).

(d) Warranty. Subject to the terms of this Section 3.2(d), Landlord warrants that the materials and workmanship comprising Landlord's Work will be free from defects or deficiencies. Any portion of Landlord's Work not conforming to the previous sentence may be considered defective.

Landlord's warranty excludes remedy for damage caused by abuse by any of the Tenant Parties or modifications not made by Landlord or any Landlord Party or improper or insufficient maintenance to the extent that such maintenance is not the responsibility of Landlord hereunder, it being understood and agreed that normal wear and tear and normal usage are not deemed defects or deficiencies. Landlord agrees that it shall, without cost to Tenant, correct any portion of Landlord's Work which is found to be defective promptly following the date that Tenant gives Landlord written notice (a "**Defect Notice**") of such defective condition, provided that the Defect Notice is delivered to Landlord on or before the date (the "**Warranty Expiration Date**") that is three hundred sixty (360) days following the substantial completion of the applicable phase of Landlord's Work, *time being of the essence*, it being understood and agreed that there shall be a separate Warranty Expiration Date for each phase of Landlord's Work. Landlord's obligations under this Section 3.2(d) shall expire on the Warranty Expiration Date and be of no further force and effect except with respect to any defects or deficiencies in Landlord's Work disclosed in any Defect Notice delivered before the Warranty Expiration Date. In addition to and notwithstanding the foregoing, Landlord hereby agrees, at no cost to Tenant, to use reasonable efforts to enforce its warranties against any contractor performing any portion of Landlord's Work and, if Landlord reasonably concludes in good faith that the cost to bring the claim and the resulting benefits to the Building and the occupants therein do not justify pursuing the warranty claim then Tenant may, but is not required to, obtain a non-exclusive assignment of such claim from Landlord and to pursue the same at Tenant's sole cost and expense. Nothing in this Section 3.2(d) shall be deemed to limit Landlord's obligations for maintenance and repair in accordance with Section 10.2 of the Lease.

(e) Remedies for Landlord's Failure to Timely Complete Phase I of Landlord's Work. Landlord shall complete Landlord's Work no later than the Substantial Completion of Tenant's Work. Notwithstanding anything to the contrary contained herein, if Phase I of Landlord's Work is not substantially complete:

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(i) by the date which is one (1) month after the Target Date (the "**Outside Date**"), subject to Tenant Delays and Force Majeure (provided that Landlord shall use commercially reasonable efforts to mitigate the impacts of Force Majeure and promptly notify Tenant of the occurrence of the same), then Tenant shall be entitled to one (1) day's abatement of Base Rent following the Rent Commencement Date for each of the first forty-five (45) days that the substantial completion of Phase I of Landlord's Work has not occurred, and two (2) day's Base Rent for each such day thereafter, until Phase I of Landlord's Work is substantially complete, and

(ii) by the date which is six (6) months after the Target Date (the "**Drop-Dead Date**"), subject to Tenant Delays and Force Majeure (provided that Landlord shall use commercially reasonable efforts to mitigate the impacts of Force Majeure and promptly notify Tenant of the occurrence of the same, and provided that such extensions for Force Majeure shall not exceed fifteen (15) months in the aggregate), then Tenant shall have the right to either (A) terminate this Lease by at least thirty (30) days' prior written notice to Landlord (provided that if substantial completion occurs within such 30 day period then Tenant's termination notice shall be null and void), or (B) substantially complete Phase I of Landlord's Work, on Landlord's behalf, in which event Landlord shall reimburse Tenant within thirty (30) days after receipt of a reasonably detailed invoice for all reasonable costs and expenses incurred by Tenant in connection therewith. Tenant's self-help rights under Section 3.2(e)(ii)(B) shall be exercised by Tenant only after Tenant has provided Landlord with notice of Tenant's intention to exercise such right (which notice shall be delivered in an envelope that conspicuously states the following in bold caps: "**TENANT NOTICE OF INTENTION TO EXERCISE SELF-HELP**") and which notice shall include an explicit statement that such notice is a notice delivered pursuant to Section 3.2(e)(ii) and Landlord's failure to perform the specified obligation will trigger the provisions of Section 3.2(e)(ii), and Landlord has failed to commence action to remedy the condition complained of within ten (10) days after its receipt of such notice (or if Landlord commences to do the act required within such ten (10) day period but fails to proceed diligently thereafter). The rights set forth in Section 3.2(e)(ii)(B) are personal to uniQure, Inc. and its Successor(s). If Landlord fails to reimburse Tenant for Tenant's costs incurred pursuant to this Section 3.2(e)(ii) within the aforementioned thirty (30) day period, then Tenant may send Landlord a notice in an envelope that conspicuously states the following in bold caps: "**TENANT NOTICE OF INTENTION TO EXERCISE OFF-SET**" and which notice shall include an explicit statement that such notice is a notice delivered pursuant to this Section 3.2(e)(ii) and describing Landlord's failure to make such reimbursement and, if Landlord fails to reimburse Tenant within ten (10) days following delivery of such notice, then Tenant may off-set such amounts, together with interest at the Default Rate from the date incurred by Tenant, against the Rent due hereunder until Tenant is paid in full.

(f) Remedies for Landlord's Failure to Timely Complete Phase II of Landlord's Work. Notwithstanding anything to the contrary contained herein, if the portions of Phase II of Landlord's Work relating to Tenant's exclusive parking spaces (the "**Parking Work**") is not substantially complete on or before the date which is six (6) months after Substantial Completion of Tenant's Work, then Tenant shall have the right to substantially complete the Parking Work, on Landlord's behalf, in which event Landlord shall reimburse Tenant within thirty (30) days after receipt of a reasonably detailed invoice for all reasonable costs and expenses incurred by Tenant in connection therewith. Tenant's self-help rights under this Section 3.2(f) shall be exercised by Tenant only after Tenant has provided Landlord with notice of Tenant's intention to exercise such right (which notice shall be delivered in an envelope that conspicuously states the following in bold caps: "**TENANT NOTICE OF INTENTION TO EXERCISE SELF-HELP**") and which notice shall include an explicit statement that such notice is a notice delivered pursuant to this Section 3.2(f) and Landlord's failure to perform the specified obligation will trigger the provisions of this Section 3.2(f), and Landlord has failed to commence action to remedy the condition complained of within ten (10) days after its receipt of such notice (or if Landlord commences to do the act required within such ten (10) day period but fails to proceed diligently

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thereafter). The provisions of this Section 3.2(f) are personal to uniQure, Inc. and its Successor(s). If Landlord fails to reimburse Tenant for Tenant's costs incurred pursuant to this Section 3.2(f) within the aforementioned thirty (30) day period, then Tenant may send Landlord a notice in an envelope that conspicuously states the following in bold caps: "**TENANT NOTICE OF INTENTION TO EXERCISE OFF-SET**" and which notice shall include an explicit statement that such notice is a notice delivered pursuant to this Section 3.2(f) and describing Landlord's failure to make such reimbursement and, if Landlord fails to reimburse Tenant within ten (10) days following delivery of such notice, then Tenant may off-set such amounts, together with interest at the Default Rate from the date incurred by Tenant, against the Rent due hereunder until Tenant is paid in full.

**3.3 Tenant's Work(a) Tenant's Plans.** In connection with the performance of the work necessary to prepare the Premises for Tenant's initial occupancy and business operations, including without limitation, the installation of all furniture and fixtures ("**Tenant's Work**"), (A) Tenant has submitted to Landlord, and Landlord has approved, a preliminary description of certain aspects of Tenant's Work attached hereto as Exhibit 3C ("**Tenant's Program**"), and (B) Tenant shall submit to Landlord for Landlord's approval (i) the name of and other reasonably requested information regarding Tenant's proposed architect, licensed structural engineer, HVAC and MEP engineers and general contractor; (ii) on or before July 15, 2013, a set of schematic plans for Tenant's proposed design of the Premises (the "**Schematic Plans**"), (iii) on or before September 30, 2013, an initial set of permit plans sufficient to permit Tenant to commence Tenant's Work ("**Permit Plans**"), and (iv) on or before October 31, 2013, a full set of construction drawings ("**Final Construction Drawings**") for Tenant's Work. The Schematic Plans, the Permit Plans and the Final Construction Drawings are collectively referred to herein as the "**Plans**." Landlord's approval of the architect, HVAC and MEP engineers and general contractor shall not be unreasonably withheld, conditioned or delayed. Landlord acknowledges that the architect, HVAC and MEP engineers and general contractor listed on Exhibit 3D attached hereto are hereby approved. Landlord's approval of the Plans shall not be

unreasonably withheld, conditioned or delayed. Landlord's approval is solely given for the benefit of Landlord and Tenant under this Section 3.3(a) and neither Tenant nor any third party shall have the right to rely upon Landlord's approval of the Plans for any other purpose whatsoever. Landlord agrees to respond to any request for approval of the Plans within ten (10) business days after receipt thereof and to respond to any re-submitted request for approval of the Plans following initial submittal of the same within three (3) business days after receipt thereof. Landlord shall cooperate with Tenant, at Tenant's sole cost and expense, in connection with Tenant's application for any state or municipal permits or approvals required in connection with the design, construction or maintenance of Tenant's Work, including signing applications therefor to the extent required of building or property owners; provided, however, in no event shall Tenant apply for any special permits or variances from the Town of Lexington without Landlord's approval, not to be unreasonably withheld, conditioned or delayed, it being understood and agreed that in no event shall any special permit or variance for which Tenant applies impose any conditions on Landlord unless approved by Landlord in its sole discretion.

(b) Landlord Delay. A "**Landlord Delay**," shall be defined as any act or omission by Landlord or any agent, employee, consultant, contractor or subcontractor of Landlord which causes an actual delay in the completion of Tenant's Work, provided that no Landlord Delay shall be deemed to occur until Tenant gives Landlord written notice of the event giving rise to such claimed Landlord Delay and a reasonable description of the same.

(c) Weekly Meetings. During the course of design and construction of Tenant's Work, Tenant shall schedule and conduct weekly meetings regarding the scheduling, progress, performance and construction of Tenant's Work. Tenant shall cause its approved architect, engineers and contractor to participate in such meetings as reasonably necessary and/or as reasonably requested by Landlord. Landlord and Landlord's agents, contractors, representatives, and lenders may attend such

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meetings and Tenant shall provide reasonably requested information thereto at such meetings. Draft requisitions (as defined in Section 3.4 below) shall be reviewed and discussed at such meetings before being submitted to Landlord. Tenant shall periodically between such meetings, at Landlord's request, provide Landlord with status updates regarding the progress of Tenant's Work. During the course of design and construction, Tenant shall cause the construction schedule for Tenant's Work to be updated periodically to reflect the actual progress of design and construction, as applicable, and shall cause such updates to be delivered to Landlord at the next weekly meeting after Tenant's receipt thereof, but such updates shall not result in or be deemed to constitute Landlord's approval of any failure to timely Substantially Complete Tenant's Work in the absence of a Landlord Delay or Force Majeure.

(d) Completion of Tenant's Work. Tenant shall Substantially Complete (hereinafter defined) Tenant's Work on or before the date that is twelve (12) months after the Term Commencement Date (the "**Outside Tenant Work Completion Date**"), provided that if Tenant is delayed in the performance of Tenant's Work by reason of a Landlord Delay or Force Majeure, the Outside Tenant Work Completion Date shall be extended by the period of time which Tenant is so delayed. For purposes hereof, Tenant's Work shall be deemed "**Substantially Complete**" and "**Substantial Completion**" shall be deemed to have occurred if Tenant has obtained a certificate of occupancy from the Town of Lexington, Massachusetts, to the extent that a certificate of occupancy is not unavailable as a result of Landlord's Work or any other work being performed outside the Premises in the Building.

(e) Cost of Tenant's Work; Priority of Work. Except for Landlord's Contribution (hereinafter defined) or as set forth in Section 3.2(a) hereof, all of Tenant's Work shall be performed at Tenant's sole cost and expense, and shall be performed in accordance with the provisions of this Lease (including, without limitation, Section 11). Landlord and Tenant acknowledge and agree that Punchlist Items relating to Phase I of Landlord's Work and Tenant's Work shall be performed concurrently. The parties shall cooperate in all commercially reasonable ways to avoid any delay in either Landlord's Work or Tenant's Work or any conflict with the performance of either Landlord's Work or Tenant's Work.

(f) Construction of Mezzanine Level. Landlord acknowledges that Tenant's Work or future Alterations may include the construction of a mezzanine level in a portion of the Premises. If Tenant constructs such a mezzanine level, Tenant acknowledges and agrees that (i) in no event shall Tenant use such space for any purpose that increases the Net Floor Area (as defined in the Town of Lexington zoning bylaw at the time of the issuance of the building permit for such work) of the Building, and (ii) such space shall be devoted exclusively to the operation and maintenance of equipment and machinery serving the Premises, such as heating, ventilating and cooling equipment, electrical and telephone facilities, fuel storage, elevator machinery or mechanical equipment. In no event shall any such mezzanine level be counted towards the measurement of the rentable square footage of the Premises hereunder.

(g) Sewer Connection. Landlord and Tenant acknowledge and agree that, as part of Tenant's Work, Tenant shall connect Tenant's exclusive sewer and wastewater discharge pipes to Landlord's existing out-flow connection to municipal sewer, subject to the terms and conditions of this Section 3. If Tenant or its contractors encounters any Hazardous Materials when making such connections, (i) Tenant shall notify Landlord immediately thereof and cease, or cause to be ceased, such work pending further direction from Landlord and (b) promptly after receipt of such notice from Tenant Landlord shall undertake the remediation of such conditions as further provided in Section 17.8 below, at its sole cost and expense such that Tenant can complete its installation without the necessity of taking any special precautions (including the remediation of soils or shipping soils off site) for Hazardous Materials. So long as Tenant is working within the area approved by Landlord in connection with its approval of such work, Landlord acknowledges that any delay in the progress of Tenant's Work resulting from remediation pursuant to the immediately preceding sentence may be a Landlord Delay. During the Term,

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Tenant may operate and shall repair and maintain such pipes and connections in accordance with the standards set forth in Section 10.1 below.

(h) Demising of Loading Dock. Landlord acknowledges that Tenant's Work or future Alterations may include the demising of the loading docks located in the Premises and dedicated to Tenant's exclusive use, subject to compliance with the terms of this Section 3 and/or 11, as applicable.

### **3.4 Landlord's Contribution.**

(a) Amount. As an inducement to Tenant's entering into this Lease, Landlord shall, subject to Section 3.4(c) below and the last sentence of this Section 3.4(a), provide to Tenant a special tenant improvement allowance equal to Seven Million Two Hundred Six Thousand Six Hundred Thirty-Nine and 30/100 Dollars (\$7,206,639.30) ("**Landlord's Contribution**") to be used by Tenant solely for hard and soft costs incurred by Tenant for Tenant's Work, provided, however, in no event shall more than five percent (5%) of Landlord's Contribution be used for such soft costs. For the purposes hereof, the cost to be so reimbursed by Landlord shall not include: (i) the cost of any of Tenant's Property (hereinafter defined), including without limitation telecommunications and computer equipment and all associated wiring and cabling, any de-mountable decorations, artwork and partitions, signs, and trade fixtures, and (ii) any fees paid to Tenant or any affiliate of Tenant.

(b) **Requisitions.** Subject to Section 3.4(c) below, Landlord shall pay Landlord's Proportion (hereinafter defined) of the cost shown on each requisition (hereinafter defined) submitted by Tenant to Landlord within thirty (30) days of submission thereof by Tenant to Landlord until the entirety of Landlord's Contribution has been exhausted. "**Landlord's Proportion**" shall be a fraction, the numerator of which is Landlord's Contribution and the denominator of which is the total price for all of Tenant's Work for the Premises (as evidenced by reasonably detailed documentation, including without limitation copies of all contracts therefor, delivered to Landlord with the requisition first submitted by Tenant), but in no event greater than one (1). A "**requisition**" shall mean written documentation (including, without limitation, invoices or bills from Tenant's contractors, vendors, service providers and consultants (collectively, "**Contractors**") and partial lien waivers and subordinations of lien in statutory form, as specified in M.G.L. Chapter 254, Section 32 for applicable contractors and service providers ("**Lien Waivers**") with respect to the prior month's requisition, and such other documentation as Landlord or any Mortgagee may reasonably request) showing in reasonable detail the costs of the item in question or of the improvements installed to date in the Premises, accompanied by reasonable evidence (including certifications executed by Tenant or Tenant's architect, subject to the architect's standard of care) that the amount of the requisition in question does not exceed the cost of the items, services and work covered by such requisition. Notwithstanding the foregoing, Tenant shall not be required to deliver Lien Waivers at the time of the first requisition, but shall deliver the Lien Waivers and evidence of payment of the first requisition in full within five (5) days following payment of Landlord's Contribution with respect to such first requisition. Landlord shall have the right, upon reasonable advance notice to Tenant, to inspect Tenant's books and records relating to each requisition in order to verify the amount thereof. Tenant shall submit requisition(s) no more often than monthly.

(c) Notwithstanding anything to the contrary herein contained: (i) Landlord shall have no obligation to advance funds on account of Landlord's Contribution more than once per month; (ii) If Tenant fails to pay to Tenant's contractors the amounts paid by Landlord to Tenant in connection with any previous requisition(s), Landlord shall thereafter have the right to have Landlord's Contribution paid directly to Tenant's contractors; (iii) Landlord shall have no obligation to pay any portion of Landlord's Contribution with respect to any requisition submitted after the date (the "**Outside Requisition Date**") which is, subject to extension to the extent that the completion of the Tenant's Work is delayed due to Force Majeure and Landlord Delay, the earlier of: (a) six (6) months after the completion of Tenant's

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Work, or (b) eighteen (18) months after the Term Commencement Date; provided, however, that if Tenant certifies to Landlord that it is engaged in a good faith dispute with any contractor, such Outside Requisition Date shall be extended while such dispute is ongoing, so long as Tenant is diligently prosecuting the resolution of such dispute; (iv) Tenant shall not be entitled to any unused portion of Landlord's Contribution; (v) Landlord's obligation to pay any portion of Landlord's Contribution shall be conditioned upon there existing no default by Tenant in its obligations under this Lease at the time that Landlord would otherwise be required to make such payment (it being understood and agreed that if Tenant cures any such defaults prior to the expiration of applicable notice and cure periods, Landlord shall thereafter make such payment); and (vi) in addition to all other requirements hereof, Landlord's obligation to pay the final five percent (5%) of Landlord's Contribution shall be subject to simultaneous delivery of all Lien Waivers relating to items, services and work performed in connection with Tenant's Work.

(d) If Landlord fails to timely pay any installment of Landlord's Contribution pursuant to this Section 3.4 (including without limitation the LULA Reimbursement pursuant to Section 3.5, below), then Tenant may send Landlord a notice in an envelope that conspicuously states the following in bold caps: "**TENANT NOTICE OF INTENTION TO EXERCISE OFF-SET**" and which notice shall include an explicit statement that such notice is a notice delivered pursuant to this Section 3.4(d) and describing Landlord's failure to make such payment and, if Landlord fails to make such payment to Tenant within ten (10) days following delivery of such notice, then Tenant may off-set against the Rent due hereunder an amount equal to such installment(s) of Landlord's Contribution which Landlord fails to timely pay, together with interest at the Default Rate from the date on which such installment was due, until (i) Tenant is paid in full for such installment, or (ii) Landlord pays the balance of such installment to Tenant.

**3.5 LULA Work.** Landlord's Work Plans include, and Landlord has prepared and delivered to Tenant (or at Tenant's direction, Tenant's general contractor) such plans relating to the work (the "**LULA Work**") necessary to install a limited use/ limited access elevator with 1,400 lb capacity (the "**LULA**"). Landlord will purchase all necessary equipment and materials and cause its contractor to perform the portion of the LULA Work that can be done prior to completion of the other items of Phase I of Landlord's Work. Upon Substantial Completion of Phase I of Landlord's Work, Landlord shall assign the subcontract for the LULA Work to Tenant's contractor and Tenant shall, at Tenant's sole cost and expense, substantially in accordance with such plans, and otherwise in accordance with Section 11 of this Lease, cause its contractor to complete the installation of the LULA. Landlord shall be responsible for all amounts due under the LULA subcontract prior to the date of such assignment. Landlord's Contribution shall be increased by an agreed upon amount (the "**LULA Reimbursement**") to reflect the estimated costs to be incurred by Tenant to complete the installation of the LULA following such assignment, which amount shall be confirmed by the parties in writing prior to Substantial Completion of Phase I of Landlord's Work, and shall be based on the costs to complete such work under the subcontract plus an additional amount for the electrical work necessary to complete such installation. Landlord shall obtain Tenant's prior written consent to the LULA subcontract, such consent not to be unreasonably withheld, conditioned or delayed.

#### **4. USE OF PREMISES**

**4.1 Permitted Uses.** During the Term, Tenant shall use the Premises only for the Permitted Uses and for no other purposes. Service and utility areas (whether or not a part of the Premises) shall be used only for the particular purpose for which they are designed.

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#### **4.2 Prohibited Uses.**

(a) Notwithstanding any other provision of this Lease, Tenant shall not use the Premises or the Building, or any part thereof, or suffer or permit the use or occupancy of the Premises or the Building or any part thereof by any of the Tenant Parties (i) in a manner which violates any of the covenants, agreements, terms, provisions and conditions of this Lease; (ii) for any unlawful purposes or in any unlawful manner; (iii) which, in the reasonable good faith judgment of Landlord (taking into account the use of the Building as a combination laboratory, research and development and office building and the Permitted Uses) shall (a) impair the appearance or reputation of the Building; (b) impair, interfere with or otherwise diminish the quality of any of the Building services, or the use of any of the Common Areas; (c) occasion discomfort, inconvenience or annoyance in any material respect, or cause any injury or damage to any other tenants or occupants of the Building or their property; or (d) cause harmful air emissions, laboratory odors or noises or any unusual or other objectionable odors, noises or emissions to emanate from the Premises taking into account the use of the Building as a combination laboratory, research and development and office building and the Permitted Uses); (iv) in a manner which is materially inconsistent with the operation and/or maintenance of the Building as a first-class combination office, research, development and laboratory facility; (v) [intentionally omitted]; or (vi) in a manner which shall increase such insurance rates on the Building or on property located therein over that applicable when Tenant first took occupancy of the Premises hereunder unless Tenant pays such increase within

thirty (30) days after demand therefor from time to time. From and after the Execution Date, Landlord shall include substantially similar provisions in any other leases at the Building and shall not enforce such provisions in a discriminatory manner.

(b) With respect to the use and occupancy of the Premises and the Common Areas, Tenant will not: (i) place or maintain any signage (except as set forth in Section 12.2 below), trash, refuse or other articles in any vestibule or entry of the Premises, on the footwalks or corridors adjacent thereto or elsewhere on the exterior of the Premises, nor obstruct any driveway, corridor, footwalk, parking area, mall or any other Common Areas; (ii) permit undue accumulations of or burn garbage, trash, rubbish or other refuse within or without the Premises; (iii) permit the parking of vehicles so as to interfere with the use of any driveway, corridor, footwalk, parking area, or other Common Areas; (iv) receive or ship articles of any kind outside of those areas reasonably designated by Landlord; (v) conduct or permit to be conducted any auction, going out of business sale, bankruptcy sale (unless directed by court order), or other similar type sale in or connected with the Premises; (vi) use the name of Landlord, or any of Landlord's affiliates in any publicity, promotion, trailer, press release, advertising, printed, or display materials without Landlord's prior written consent, which shall not be unreasonably withheld, conditioned or delayed; or (vii) except in connection with Alterations (hereinafter defined) approved by Landlord or otherwise not requiring Landlord's approval, cause or permit any hole to be drilled or made in any part of the Building.

## 5. RENT; ADDITIONAL RENT

**5.1 Base Rent.** During the Term, Tenant shall pay to Landlord Base Rent in equal monthly installments, in advance and without demand on the first day of each month for and with respect to such month. Unless otherwise expressly provided herein, the payment of Base Rent, additional rent and other charges reserved and covenanted to be paid under this Lease with respect to the Premises (collectively, "**Rent**") shall commence on the Rent Commencement Date, and shall be prorated for any partial months. Rent shall be payable to Landlord or, if Landlord shall so direct in writing, to Landlord's agent or nominee, in lawful money of the United States which shall be legal tender for payment of all debts and dues, public and private, at the time of payment.

### 5.2 Operating Costs.

(a) "**Operating Costs**" shall mean all reasonable costs incurred and expenditures of whatever nature made by Landlord in the operation, management, repair, replacement, maintenance and

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insurance of the Property or allocated to the Property, including without limitation any costs for utilities supplied to exterior areas of the Property and the Common Areas, and any costs for repair and replacements, cleaning and maintenance of exterior areas and the Common Areas, related equipment, facilities and appurtenances and HVAC equipment, a commercially reasonable management fee paid to Landlord's property manager not to exceed 4% of gross rents from the Building, and the cost of operating any amenities available to all tenants of the Property as of the date of this Lease. Operating Costs shall not include Excluded Costs (hereinafter defined) and shall be calculated in accordance with sound accounting principles and practices.

(b) "**Excluded Costs**" shall be defined as (i) any mortgage charges (including interest, principal, points and fees, including attorneys' fees); (ii) brokerage commissions; (iii) salaries of executives and owners not directly employed in the management/operation of the Property or above the level of property manager; (iv) the cost of work done by Landlord or services for any particular tenant; (v) the cost of items which, by generally accepted accounting principles, would be capitalized on the books of Landlord or are otherwise not properly chargeable against income, except to the extent such capital item is (A) required by any Legal Requirements enacted after the Commencement Date, or (B) reasonably projected to reduce Operating Costs, but only to the extent in any fiscal year of the savings in Operating Costs actually resulting from such capital expenditure, in each case amortized over its useful life as determined in accordance with GAAP; (vi) the costs of Landlord's Work (including any costs to correct defects in the Landlord's Work pursuant to Section 3.2 above) and any contributions made by Landlord to any tenant of the Property in connection with the build-out of its premises; (vii) franchise or income taxes imposed on Landlord; (viii) costs paid directly by individual tenants to suppliers, including tenant electricity, telephone and other utility costs; (ix) increases in premiums for insurance when such increase is caused by the use of the Building by Landlord or any other tenant of the Building; (x) maintenance and repair of capital items not a part of the Building or the Property; (xi) depreciation of the Building; (xii) costs relating to maintaining Landlord's existence as a corporation, partnership or other entity; (xiii) advertising and other fees and costs incurred in procuring tenants; (xiv) the cost of any items for which Landlord is reimbursed by insurance, condemnation awards, refund, rebate or otherwise, and any expenses for repairs or maintenance to the extent covered by warranties, guaranties and service contracts; (xv) costs incurred in connection with any disputes between Landlord and its employees, between Landlord and Building management, or between Landlord and other tenants or occupants; (xvi) accounting fees; (xvii) fines and penalties; (xviii) costs and expenses of investigating, monitoring or remediating existing hazardous materials on, under or about the Building or the Property; (xix) costs of selling, syndicating, financing or refinancing any portion of the Property and/or of Landlord's interest therein (including interest, principal, points and fees, interest on debt or amortization payments on any mortgage or deed to secure debt and rental under any ground lease, master lease or other underlying lease, and other debt costs, if any); (xx) charitable or political contributions; (xxi) all items and services for which Tenant is separately charged, reimburses Landlord or pays third persons; (xxii) reserves of any kind (including without limitation reserves for bad debts or rent loss) or any bad debt loss or rent loss; (xxiii) the costs of goods and services provided by affiliates of Landlord, to the extent only that the costs of such goods and/or services exceed the market rate for goods or services of comparable quality provided by unrelated third parties; (xxiv) costs incurred to develop and construct additional structures on the Property (including without limitation any Future Development), (xxv) costs appropriately allocable to any other building (any Operating Costs that are incurred with respect to the Building and any other building located on the Property or another property shall be allocated between such buildings based on the rentable square footage of such buildings unless another method more equitably reflects the benefit of such costs), (xxvi) the cost of acquiring (as opposed to leasing) sculptures, paintings and other objects of art (provided, however, that any costs of leasing sculptures, paintings and other objects of art shall be customary for first-class buildings in the vicinity of the Property); (xxvii) the cost of advertising or promotion of (A) the Property or any part thereof or (B) any operations at the Property; (xxviii) Landlord's general overhead.

(c) Payment of Operating Costs. Tenant shall pay to Landlord, as additional rent,

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**Tenant's Share of Operating Costs.** Landlord shall make a good faith estimate of Tenant's Share of Operating Costs for any fiscal year or part thereof during the term and notify Tenant of such estimate no later than thirty (30) days prior to each fiscal year, and Tenant shall pay to Landlord, on the Rent Commencement Date and on the first (1st) day of each calendar month thereafter, an amount equal to Tenant's Share of Operating Costs for such fiscal year and/or part thereof divided by the number of months therein. Upon at least thirty (30) days' notice, Landlord may estimate and re-estimate Tenant's Share of Operating Costs and deliver a copy of the estimate or re-estimate to Tenant, provided that no more than one re-estimate may be made in any given fiscal year. Thereafter, the monthly installments of Tenant's Share of Operating Costs shall be appropriately adjusted in accordance with the estimations so that, by the end of the fiscal year in question, Tenant shall have paid all of Tenant's Share of Operating Costs as estimated by Landlord. Any amounts paid based on such an estimate shall be subject to reconciliation as herein provided when actual Operating Costs are available for each fiscal year.

(d) Annual Reconciliation. Landlord shall, within one hundred twenty (120) days after the end of each fiscal year, deliver to Tenant a reasonably detailed statement of the actual amount of Operating Costs for such fiscal year ("Year End Statement"). Failure of Landlord to provide the Year End Statement within the time prescribed shall not relieve Tenant from its obligations hereunder, provided, however, Landlord shall be deemed to have waived any costs actually incurred but not billed to Tenant within two (2) years after the end of the fiscal year in which such cost was incurred by Landlord. If the total of such monthly remittances on account of any fiscal year is greater than Tenant's Share of Operating Costs actually incurred for such fiscal year, then, provided no Event of Default nor any event which, with the passage of time and/or the giving of notice would constitute an Event of Default, is then continuing (it being understood and agreed that if Tenant cures any default(s) within the applicable cure period(s) provided in Section 20 below, then Tenant shall thereafter be entitled to take such credit), Tenant may credit the difference against the next installment(s) of additional rent on account of Operating Costs due hereunder, except that if such difference is determined after the end of the Term, Landlord shall refund such difference to Tenant within thirty (30) days after such determination to the extent that such difference exceeds any amounts then due from Tenant to Landlord. If the total of such remittances is less than Tenant's Share of Operating Costs actually incurred for such fiscal year, Tenant shall pay the difference to Landlord, as additional rent hereunder, within thirty (30) days of Tenant's receipt of an invoice therefor. Landlord's estimate of Operating Costs for the next fiscal year shall be based upon the Operating Costs actually incurred for the prior fiscal year as reflected in the Year-End Statement plus a reasonable adjustment based upon estimated increases in Operating Costs, if any. The provisions of this Section 5.2(d) shall survive the expiration or earlier termination of this Lease.

(e) Partial Years. If the Rent Commencement Date or the Expiration Date occurs in the middle of a fiscal year, Tenant shall be liable for only that portion of the Operating Costs with respect to such fiscal year within the Term.

(f) Gross-Up. If, during any fiscal year, less than 95% of the Building is occupied by tenants or if Landlord was not supplying all tenants with the services being supplied to Tenant hereunder, actual Operating Costs incurred shall be reasonably extrapolated by Landlord on an item-by-item basis to the reasonable Operating Costs that would have been incurred if the Building was 95% occupied and such services were being supplied to all tenants, and such extrapolated Operating Costs shall, for all purposes hereof, be deemed to be the Operating Costs for such fiscal year. This "gross up" treatment shall be applied only with respect to variable Operating Costs arising from services provided to Common Areas or to space in the Building being occupied by tenants (which services are not provided to vacant space, i.e. such Operating Costs vary with occupancy, or may be provided only to some tenants) in order to allocate equitably such variable Operating Costs to the tenants receiving the benefits thereof. Furthermore, Tenant acknowledges that the costs of cleaning, maintaining and repairing the Common Hallway shall be allocated to Tenant and the tenant(s) of the Unleased Space based on rentable square feet.

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(g) Audit. Provided there is no Event of Default nor any event which, with the passage of time and/or the giving of notice would constitute an Event of Default, Tenant may, upon at least ten (10) days' prior written notice, inspect or audit Landlord's records relating to Operating Costs and/or Taxes for any periods of time within the previous fiscal year before the audit or inspection (it being understood that if Tenant shall cure any such default within applicable notice and/or cure periods provided in Section 20.1 below, then Tenant shall thereafter be entitled to perform such inspection or audit). Landlord shall provide Tenant with access to such records at a location within the Greater Boston area in accordance with this Section 5.2(g) within ten (10) days after receipt of notice from Tenant. However, no audit or inspection shall extend to periods of time before the Rent Commencement Date. If Tenant fails to object to the calculation of Tenant's Share of Operating Costs and/or Taxes on the Year-End Statement within ninety (90) days after such statement has been delivered to Tenant and/or fails to complete any such audit or inspection within sixty (60) days after Landlord's records are made available to Tenant in accordance with this Section 5.2(g), then Tenant shall be deemed to have waived its right to object to the calculation of Tenant's Share of Operating Costs and/or Taxes, as the case may be, for the year in question and the calculation thereof as set forth on such statement shall be final. Tenant's audit or inspection shall be conducted only at Landlord's offices or the offices of Landlord's property manager at a location within the Greater Boston area during business hours reasonably designated by Landlord. Tenant shall pay the cost of such audit or inspection, provided, however, that if such audit discloses that Tenant has been overcharged by more than five percent (5%), Landlord shall reimburse Tenant for Tenant's reasonable out-of-pocket costs incurred in connection with such audit. Tenant may not conduct an inspection or have an audit performed more than once during any fiscal year. If such inspection or audit reveals that an error was made in the calculation of Tenant's Share of Operating Costs or Taxes previously charged to Tenant, then, provided there is no Event of Default nor an event which, with the passage of time and/or the giving of notice would constitute an Event of Default, Tenant may credit the difference against the next installment of additional rent on account of Operating Costs or Taxes, as the case may be, due hereunder (it being understood that if Tenant shall cure any such default within applicable notice and/or cure periods provided in Section 20.1 below, then Tenant shall thereafter be entitled to take such credit), except that if such difference is determined after the end of the Term, Landlord shall refund such difference to Tenant within thirty (30) days after such determination to the extent that such difference exceeds any amounts then due from Tenant to Landlord. If such inspection or audit reveals an underpayment by Tenant, then Tenant shall pay to Landlord, as additional rent hereunder, any underpayment of any such costs, after deducting the reasonable out of pocket costs of such inspection or audit, within thirty (30) days after such underpayment is determined. Tenant shall maintain the results of any such audit or inspection confidential and shall not be permitted to use any third party to perform such audit or inspection, other than an independent firm of certified public accountants (A) reasonably acceptable to Landlord, (B) which is not compensated on a contingency fee basis or in any other manner which is dependent upon the results of such audit or inspection, and (C) which executes Landlord's standard confidentiality agreement whereby it shall agree to maintain the results of such audit or inspection confidential. The provisions of this Section 5.2(g) shall survive the expiration or earlier termination of this Lease.

### 5.3 Taxes.

(a) "Taxes" shall mean the real estate taxes and other taxes, levies and assessments imposed upon the Building and the Land, and upon any personal property of Landlord used in the operation thereof, or on Landlord's interest therein or such personal property; charges, fees and assessments for transit, housing, police, fire or other services or purported benefits to the Building and the Land (including without limitation any community preservation assessments) that are charged on the real estate tax bills issued by the Town of Lexington to all property owners in the Town; service or user payments in lieu of taxes; and any and all other taxes, levies, betterments, assessments and charges arising from the ownership, leasing, operation, use or occupancy of the Building and the Land or based upon rentals derived therefrom, which are or shall be imposed by federal, state, county, municipal or other governmental authorities. From and after substantial completion of any occupiable improvements

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constructed as part of a Future Development, if such improvements are not separately assessed, Landlord shall reasonably allocate Taxes between the Building and such improvements and the land area associated with the same and shall, as soon as possible thereafter, seek to have such building separately assessed. From and after the issuance of a building permit for construction of any occupiable improvements constructed as part of a Future Development, Taxes shall not include any Taxes assessed on such improvements under construction to the extent evidenced by the Town of Lexington's assessors' records. Taxes shall not include (i) any inheritance, estate, succession, gift, franchise, rental, income or profit tax, capital stock tax, capital levy or excise, or any income taxes arising out of or related to

the ownership and operation of the Building and the Land, provided, however, that any of the same and any other tax, excise, fee, levy, charge or assessment, however described, that may in the future be levied or assessed as a substitute for or an addition to, in whole or in part, any tax, levy or assessment which would otherwise constitute Taxes, whether or not now customary or in the contemplation of the parties on the Execution Date of this Lease, shall constitute Taxes, but only to the extent calculated as if the Building and the Land were the only real estate owned by Landlord, (ii) any interest or penalties resulting from the late payment of Taxes by Landlord (except to the extent due to Tenant's failure to make timely payments), (iii) any taxes assessed against air or other development rights; (iv) any environmental assessments, charges or liens arising in connection with the remediation of Hazardous Materials from the Building or Property (provided that the foregoing shall not be deemed to derogate from Tenant's obligations set forth in Section 17 below with respect to Hazardous Materials); costs or fees payable to public authorities in connection with any future construction of additional buildings or similar improvements on the Property (including any such fees for transit, housing, schools, open space, child care, arts programs, traffic mitigation measures, environmental impact reports and traffic studies); reserves for future Taxes; and any personal property taxes attributable to sculptures, paintings or other objects of art. "Taxes" shall also include reasonable expenses (including without limitation legal and consultant fees) of tax abatement or other proceedings contesting assessments or levies.

(b) **"Tax Period"** shall be any fiscal/tax period in respect of which Taxes are due and payable to the appropriate governmental taxing authority (i.e., as mandated by the governmental taxing authority), any portion of which period occurs during the Term of this Lease.

(c) **Payment of Taxes.** Tenant shall pay to Landlord, as additional rent, Tenant's Building Share of taxes relating to or allocable to the Building and Tenant's Share of Taxes relating to or allocable to the Land. At least thirty (30) days prior to the Rent Commencement Date, Landlord shall notify Tenant of Landlord's good faith estimate of the Taxes to be due by Tenant for the Tax Period in which the Rent Commencement Date occurs, and Tenant shall pay to Landlord, on the Rent Commencement Date and on the first (1<sup>st</sup>) day of each calendar month thereafter, an amount equal to Landlord's estimate of Tenant's Share of Taxes for such Tax Period or part thereof divided by the number of months therein. Upon at least thirty (30) days' notice, Landlord may estimate and re-estimate Tenant's Share of Taxes and deliver a copy of the estimate or re-estimate to Tenant, provided that Landlord shall not re-estimate Tenant's Share of Taxes more than one time in any fiscal year. Thereafter, the monthly installments of Tenant's Share of Taxes shall be appropriately adjusted in accordance with the estimations so that, by the end of the Tax Period in question, Tenant shall have paid all of Tenant's Share of Taxes as estimated by Landlord. Any amounts paid based on such an estimate shall be subject to adjustment as herein provided when actual Taxes are available for each Tax Period. If the total of such monthly remittances is greater than Tenant's Share of Taxes actually due for such Tax Period, then, provided no Event of Default nor any event which, with the passage of time and/or the giving of notice would constitute an Event of Default is then continuing (it being understood and agreed that if Tenant cures any default(s) within the applicable cure period(s) provided in Section 20 below, then Tenant shall thereafter be entitled to take such credit), Tenant may credit the difference against the next installment(s) of additional rent on account of Taxes due hereunder, except that if such difference is determined after the end of the Term, Landlord shall refund such difference to Tenant within thirty (30) days after such determination to the extent that such difference exceeds any amounts then due from Tenant to Landlord. If the total of such remittances is less than Tenant's Share of Taxes actually due for such Tax Period,

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Tenant shall pay the difference to Landlord, as additional rent hereunder, within thirty (30) days of Tenant's receipt of an invoice therefor. Landlord's estimate for the next Tax Period shall be based upon actual Taxes for the prior Tax Period plus a reasonable adjustment based upon estimated increases in Taxes. The provisions of this Section 5.3(c) shall survive the expiration or earlier termination of this Lease.

(d) **Abatements.**

(i) Landlord may at any time contest any valuation of the Land, the Building or the Premises, or any tax rate, or the amount of any Taxes, by legal proceedings or in such other manner as it may deem suitable. If Tenant desires to institute such a contest, Tenant shall notify Landlord of such desire on or before the date which is thirty (30) days before the deadline to contest the same. Unless Landlord notifies Tenant at least ten (10) business days before such deadline that Landlord will institute such a contest, then, provided that during such real estate tax year Tenant's Share is at least fifty percent (50%), Tenant shall have the right during or after the Term of this Lease to contest or review any valuation of the Building or the Premises, or any tax rate, of the amount of any Taxes for such real estate tax year, by legal proceedings or in such other manner as it may deem suitable. If Tenant elects to institute such a proceeding, Tenant shall conduct it in the name of Tenant, provided that if any such proceeding which Tenant elects to institute must be prosecuted in the name of Landlord, Landlord shall permit Tenant to institute and prosecute it in the name of Landlord as provided above, but Landlord shall not settle any proceeding so instituted by Tenant without Tenant's prior written approval in each instance. If any such proceeding is instituted in the name of Tenant, Tenant shall not settle such proceeding without Landlord's prior written approval in each instance, which approval shall not be unreasonably withheld. If Tenant institutes such a proceeding, Tenant (A) shall prosecute it diligently and in good faith at all times, (B) shall prosecute it at its sole cost and expense (subject to reimbursement as provided below in this Section 5.3(d)), (C) shall periodically advise Landlord as to the status thereof, and (D) shall not abandon the same without first offering to Landlord the right to prosecute the same (and in the event that Landlord elects to continue such proceeding, Tenant shall promptly assign and turn over to Landlord the control of such proceeding, and thereafter Tenant shall have no further liability or responsibility in connection therewith). Landlord (at no cost to Landlord) shall cooperate with Tenant to the extent reasonably necessary to enable Tenant to institute and prosecute such proceeding including, without limitation, providing all information and documents reasonably requested by Tenant, executing all documents necessary to accomplish the foregoing, and making such appearances as Tenant may reasonably request.

(ii) If Landlord or Tenant obtains a refund or abatement of Taxes, (A) the parties shall first be entitled to receive reimbursement from any refund or abatement for all expenses, including reasonable attorney's fees, incurred by it in connection with obtaining such refund or abatement, and (B) then, if Tenant has paid Taxes or estimated Taxes for the period for which the refund has paid Taxes or estimated Taxes for the period for which the refund or abatement was granted, Tenant shall be entitled to receive Tenant's Share of the abatement (with interest, if any, paid by the Governmental Authority on such abatement), adjusted for any period for which Tenant had made a partial payment.

(iii) Appropriate credit against Taxes (or payments to Tenant following the termination this Lease) shall be given for any refund obtained by reason of a reduction in any Taxes by the assessors or the administrative, judicial or other governmental agency responsible therefor after deduction of Landlord's expenditures for reasonable legal fees and for other reasonable expenses incurred in obtaining the Tax refund. If Tenant provides Landlord with reasonable evidence that any exemption from real property taxes for the Property is due to any Tax Increment Financing Agreement ("**TIFA**") entered into by Tenant and the Town of Lexington, then the benefit of such exemption shall be allocated entirely to Tenant.

(e) **Part Years.** If the Rent Commencement Date or the Expiration Date occurs in the

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middle of a Tax Period, Tenant shall be liable for only that portion of the Taxes, as the case may be, with respect to such Tax Period within the Term.



#### 5.4 Late Payments.

(a) Any payment of Rent due hereunder not paid when due shall bear interest for each month or fraction thereof from the due date until paid in full at the annual rate of twelve percent (12%), or at any applicable lesser maximum legally permissible rate for debts of this nature (the “**Default Rate**”). Notwithstanding the foregoing, Landlord shall not charge such interest with respect to the first late payment in any twelve (12) month period so long as received by Landlord within five (5) business days after the due date therefor.

(b) Additionally, if Tenant fails to make any payment within five (5) days after the due date therefor, Landlord may charge Tenant a fee, which shall constitute liquidated damages, equal to One Thousand and NO/100 Dollars (\$1,000.00) for each such late payment. Notwithstanding the foregoing, Landlord shall not charge such fee with respect to the first late payment in any twelve (12) month period so long as received by Landlord within five (5) business days after the due date therefor.

(c) For each Tenant payment check to Landlord that is returned by a bank for any reason, Tenant shall pay a returned check charge equal to the amount as shall be customarily charged by Landlord’s bank at the time.

(d) Money paid by Tenant to Landlord shall be applied to Tenant’s account in the following order: first, to any unpaid additional rent, including without limitation late charges, returned check charges, legal fees and/or court costs chargeable to Tenant hereunder; and then to unpaid Base Rent.

(e) The parties agree that the late charge referenced in Section 5.4(b) represents a fair and reasonable estimate of the costs that Landlord will incur by reason of any late payment by Tenant, and the payment of late charges and interest are distinct and separate in that the payment of interest is to compensate Landlord for the use of Landlord’s money by Tenant, while the payment of late charges is to compensate Landlord for Landlord’s processing, administrative and other costs incurred by Landlord as a result of Tenant’s delinquent payments. Acceptance of a late charge or interest shall not constitute a waiver of Tenant’s default with respect to the overdue amount or prevent Landlord from exercising any of the other rights and remedies available to Landlord under this Lease or at law or in equity now or hereafter in effect.

(f) If Tenant during any six (6) month period shall be more than five (5) days delinquent in the payment of any regular monthly installment of Base Rent, Operating Costs or Taxes on three (3) or more occasions, then, notwithstanding anything herein to the contrary, Landlord may, by written notice to Tenant, elect to require Tenant to pay all Base Rent and Additional Rent on account of Operating Costs and Taxes quarterly in advance. Such right shall be in addition to and not in lieu of any other right or remedy available to Landlord hereunder or at law on account of Tenant’s default hereunder.

**5.5 No Offset; Independent Covenants; Waiver.** Rent shall be paid without notice or demand, and, except as expressly set forth herein, without setoff, counterclaim, defense, abatement, suspension, deferment, reduction or deduction. **TENANT HEREBY ACKNOWLEDGES AND AGREES THAT THE OBLIGATIONS OF TENANT HEREUNDER SHALL BE SEPARATE AND INDEPENDENT COVENANTS AND AGREEMENTS, THAT RENT SHALL CONTINUE TO BE PAYABLE IN ALL EVENTS AND THAT THE OBLIGATIONS OF TENANT HEREUNDER SHALL CONTINUE UNAFFECTED, UNLESS THE REQUIREMENT TO PAY OR PERFORM THE SAME SHALL HAVE BEEN ABATED OR TERMINATED PURSUANT TO AN EXPRESS PROVISION OF THIS LEASE. LANDLORD AND TENANT EACH**

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**ACKNOWLEDGES AND AGREES THAT THE INDEPENDENT NATURE OF THE OBLIGATIONS OF TENANT HEREUNDER REPRESENTS FAIR, REASONABLE, AND ACCEPTED COMMERCIAL PRACTICE WITH RESPECT TO THE TYPE OF PROPERTY SUBJECT TO THIS LEASE, AND THAT THIS AGREEMENT IS THE PRODUCT OF FREE AND INFORMED NEGOTIATION DURING WHICH BOTH LANDLORD AND TENANT WERE REPRESENTED BY COUNSEL SKILLED IN NEGOTIATING AND DRAFTING COMMERCIAL LEASES IN MASSACHUSETTS, AND THAT THE ACKNOWLEDGEMENTS AND AGREEMENTS CONTAINED HEREIN ARE MADE WITH FULL KNOWLEDGE OF THE HOLDING IN WESSON V. LEONE ENTERPRISES, INC., 437 MASS. 708 (2002). SUCH ACKNOWLEDGEMENTS, AGREEMENTS AND WAIVERS BY TENANT ARE A MATERIAL INDUCEMENT TO LANDLORD ENTERING INTO THIS LEASE.**

**5.6 Survival.** Any obligations under this Section 5 which shall not have been paid at the expiration or earlier termination of the Term shall survive such expiration or earlier termination and shall be paid when and as the amount of same shall be determined and be due.

#### 6. EXPANSION RIGHTS

##### 6.1 Right of Second Offer to Lease.

(a) Right of Second Offer. Landlord and Tenant acknowledge and agree that (i) the space shown on the plan attached hereto as Exhibit 1F (the “**ROSO Space**”) is currently leased to Quanterix, a third party tenant, (ii) the space shown as “AVAILABLE” on the plan attached hereto as Exhibit 1G (the “**Unleased Space**”) is currently unleased, (iii) Landlord has the right to grant to the initial future tenant of all or at least 30,000 rentable square feet of the Unleased Space (the “**Initial Tenant**”) a right of first offer to lease the ROSO Space on whatever terms and conditions Landlord determines in its sole discretion, and (iv) if such Initial Tenant refuses, or is deemed to have refused, to accept such right of first offer, then, subject to the provisions of this Section 6.1, and provided that (A) as of the date of the ROSO Notice (hereinafter defined), there is no Event of Default then continuing, (B) as of the date of the ROSO Notice, Tenant meets the Occupancy Threshold, then Tenant shall have a one-time right of second offer to lease the ROSO Space in its as-is condition, broom clean, free of personal property and decommissioned in accordance with reasonable industry standards, at then-fair market rent (based on such condition) (the “**ROSO FMR**”), for a term commencing on the date immediately after the date on which the current tenant of the ROSO Space vacates and surrenders the same (and co-terminus with the Term hereof), and otherwise upon the terms and conditions specified in the ROSO Notice. The provisions of this Section 6.1 shall terminate on the first to occur of the leasing of the ROSO Space to Initial Tenant or Tenant in accordance with this Section 6.1(a) or April 1, 2014.

(b) Offer and Acceptance Procedures for Right of Second Offer.

(i) After Landlord determines, in its reasonable judgment, that the ROSO Space is available for lease to Tenant and all of the preconditions to the right of second offer granted to Tenant in this Section 6.1, have been met, Landlord shall deliver to Tenant a written notice offering to lease the ROSO Space to Tenant upon the terms and conditions set forth herein (the “**ROSO Notice**”). Tenant then shall have ten (10) days after receipt of the ROSO Notice to notify Landlord in writing whether Tenant will exercise its right to lease the ROSO Space upon the terms and conditions described in the ROSO Notice. If Tenant fails to notify Landlord in writing within such 10- day period that Tenant accepts the offer contained in the ROSO Notice, or if Tenant refuses in writing the offer contained in the ROSO Notice, Landlord shall have the right to lease the ROSO Space to any tenant on whatever terms and conditions Landlord may decide in its sole discretion.

(ii) If Tenant timely notifies Landlord of its desire to lease the ROSO Space pursuant to this Section 6.1 (such notice, the “**ROSO Acceptance**”), Landlord shall submit to Tenant, and Tenant shall execute and deliver to Landlord within thirty (30) days of receipt thereof, a reasonable form of lease amendment which incorporates all of the terms and conditions set forth in the ROSO Notice, including without limitation Landlord’s reasonable determination of the ROSO FMR therefor (or, if applicable, the ROSO FMR determined in accordance with Section 6.1(b)(iii) below). Landlord and Tenant shall reasonably diligently negotiate such lease amendment in good faith. If the determination of the ROSO FMR is to be determined in accordance with Section 6.1(b)(iii) below and the ROSO FMR has not been determined prior to the expiration of such 30-day period, then the parties shall execute the lease amendment with the base rent amounts blank and deliver such executed originals into escrow with Landlord’s counsel. If Tenant fails to execute and deliver the lease amendment within said thirty (30) day period, *time being of the essence*, then Tenant’s right to lease the ROSO Space shall terminate and shall be null and void, and, subject to Section 6.2 below, Landlord shall have no further obligation to lease the ROSO Space to Tenant and may lease any or all of the ROSO Space to another party upon such terms and conditions as Landlord may deem appropriate, free and clear of any rights in favor of Tenant contained herein.

(iii) If Tenant timely provides the ROSO Acceptance and indicates in such ROSO Acceptance that Tenant (A) rejects Landlord’s determination of ROSO FMR, (B) desires to submit the matter to arbitration, and (C) identifies Tenant’s Appraiser, then the ROSO FMR shall be determined in accordance with the procedure set forth in this Section 6.1(b)(iii). In such event, within five (5) days after receipt by Landlord of Tenant’s ROSO Acceptance, Landlord shall notify Tenant in writing of Landlord’s Appraiser. Landlord’s Appraiser and Tenant’s Appraiser shall then jointly select the Third Appraiser within eight (8) days of their appointment. All of the brokers/appraisers selected meet the requirements set forth in Section 1.2(c) above. Landlord’s Appraiser and Tenant’s Appraiser shall deliver their determinations of the ROSO FMR to the Third Appraiser within five (5) days of the appointment of the Third Appraiser and the Third Appraiser shall render his or her decision within eight (8) days after receipt of both of the other two determinations of the ROSO FMR. The Third Appraiser’s decision shall be binding on both Landlord and Tenant. Each party shall bear the cost of its own appraiser and the cost of the Third Appraiser shall be paid by the party whose determination is not selected. If such determination is rendered after the 30-day period described in Section 6.1(b)(ii) above, then Landlord and Tenant shall jointly instruct Landlord’s counsel to insert into the escrowed amendments the ROSO FMR determined in accordance with this Section 6.1(b)(iii) and to release such completed amendments from escrow.

## 6.2 Right of First Offer to Lease.

(a) **Right of First Offer.** Subject to the provisions of this Section 6.2, from and after the later to occur of (A) July 1, 2014, and (B) the date on which the Building is 100% leased (such later date, the “**ROFO Date**”) and provided that (i) as of the date of the ROFO Notice (hereinafter defined), there is no Event of Default then continuing, (ii) as of the date of the ROFO Notice, Tenant meets the Occupancy Threshold, and (iii) as of the date on which the Available Space is expected to be delivered to Tenant, there remain at least five (5) years left in the Term (it being understood and agreed that Tenant shall have the right to elect to unconditionally exercise its option to extend the Term pursuant to Section 1.2 above, if any such right remains in force, if necessary to meet this precondition (provided that the determination of the Extension Term Base Rent shall occur as if Tenant provided its Extension Notice twelve (12) months prior to the expiration of the then-current term), Tenant shall have a continuous right of first offer to lease the other rentable areas of the Building (the “**ROFO Space**”) if, as and when the same shall become available for lease (as reasonably determined by Landlord, the “**Available Space**”), in their as-is condition, broom clean, free of personal property and decommissioned in accordance with reasonable industry standards, at then-fair market rent (based on such condition) (“**ROFO FMR**”), for a

term co-terminus with the Term hereof, and otherwise upon the terms and conditions specified in the ROFO Notice. Tenant’s right of first offer under this Section 6.2 is further subject to all extension rights existing as of the ROFO Date.

### (b) **Offer and Acceptance Procedures for Right of First Offer.**

(i) After Landlord reasonably determines that the Available Space is available for lease (meaning that it is or shall be available for lease within the next 12 month period) and all of the preconditions to the right of first offer granted to Tenant in this Section 6.2, have been met, Landlord shall deliver to Tenant a written notice offering to lease the Available Space to Tenant upon the terms and conditions set forth herein (the “**ROFO Notice**”). Tenant then shall have ten (10) days after receipt of the ROFO Notice to notify Landlord in writing whether Tenant will exercise its right to lease the Available Space upon the terms and conditions described in the ROFO Notice. If Tenant fails to notify Landlord in writing within such 10-day period that Tenant accepts the offer contained in the ROFO Notice, or if Tenant refuses in writing the offer contained in the ROFO Notice, Landlord shall have the right to lease the Available Space to any third party tenant on the terms of the ROFO Notice provided that the net effective rent may be ten percent (10%) less than the net effective rent set forth in the ROFO Notice). As used herein, the term “**net effective rent**” shall mean the net present value of the rent, additional rent, and other charges that would be payable to Landlord under the terms of any proposed lease for and with respect to that portion of the term of the proposed lease equal to the period from the commencement date of such proposed lease through the expiration date, taking into account any construction allowance, the cost of any leasehold improvements proposed to be performed by Landlord, any free rent, and any other monetary inducements payable by Landlord under such proposed lease. If Landlord does not execute a lease with a third party with respect to the Available Space in question within twelve (12) months after the date on which Tenant refuses or is deemed to have refused the offer set forth in the applicable ROFO Notice, or if Landlord desires to offer the applicable Available Space on material terms different than previously described in the ROFO Notice (subject to the proviso regarding net effective rent set forth above), then Landlord must deliver a new ROFO Notice to Tenant in accordance with Section 6.2(a) above.

(ii) If Tenant timely notifies Landlord of its desire to lease the Available Space pursuant to this Section 6.2 (such notice, the “**ROFO Acceptance**”), Landlord shall submit to Tenant, and Tenant shall execute and deliver to Landlord within forty-five (45) days of receipt thereof, a reasonable form of lease amendment which incorporates all of the terms and conditions set forth in the ROFO Notice. Landlord and Tenant shall reasonably diligently negotiate such lease amendment in good faith. If the determination of the ROFO FMR is to be determined in accordance with Section 6.2(b)(iii) below and the ROFO FMR has not been determined prior to the expiration of such 45-day period, then the parties shall execute the lease amendment with the base rent amounts blank and deliver such executed originals into escrow with Landlord’s counsel. Notwithstanding anything to the contrary, the failure by either party (or both parties) to execute such lease amendment shall not be deemed to nullify, cancel, modify or otherwise affect Tenant’s binding acceptance (as evidenced by the ROFO Acceptance) of the offer set forth in the ROFO Notice.

(iii) If Tenant timely provides the ROFO Acceptance and indicates in such ROFO Acceptance that Tenant (A) rejects Landlord’s determination of ROFO FMR, (B) desires to submit the matter to arbitration, and (C) identifies Tenant’s Appraiser, then the ROFO FMR shall be determined in accordance with the procedure set forth in this Section 6.2(b)(iii). In such event, within five (5) days after receipt by Landlord of Tenant’s ROFO Acceptance,

Landlord shall notify Tenant in writing of Landlord's Appraiser. Landlord's Appraiser and Tenant's Appraiser shall then jointly select the Third Appraiser within ten (10) days of their appointment. All of the brokers/appraisers selected meet the requirements set forth in Section 1.2(c) above. Landlord's Appraiser and Tenant's Appraiser shall deliver

their determinations of the ROFO FMR to the Third Appraiser within five (5) days of the appointment of the Third Appraiser and the Third Appraiser shall render his or her decision within ten (10) days after receipt of both of the other two determinations of the ROFO FMR. The Third Appraiser's decision shall be binding on both Landlord and Tenant. Each party shall bear the cost of its own appraiser and the cost of the Third Appraiser shall be paid by the party whose determination is not selected. If such determination is rendered after the 45-day period described in Section 6.2(b)(ii) above, then Landlord and Tenant shall jointly instruct Landlord's counsel to insert into the escrowed amendments the ROFO FMR determined in accordance with this Section 6.1(b)(iii) and to release such completed amendments from escrow.

**6.3 Termination of Rights.** All rights of Tenant under this Section 6 shall terminate upon the expiration or earlier termination of the term of this Lease.

**6.4 Rights Personal to Tenant.** Tenant may not assign, mortgage, pledge, encumber or otherwise transfer its interest or rights under this Section 6 other than to an assignee of Tenant, and any such purported transfer or attempt to transfer shall be void and without effect, shall terminate Tenant's rights under this Section 6, and shall constitute an Event of Default under this Lease.

**6.5 Time is of the Essence. Time is of the essence with respect to all aspects of this Section 6.**

## **7. LETTER OF CREDIT**

**7.1 Amount.** Contemporaneously with the execution of this Lease, Tenant shall deliver either (i) cash in the amount specified in the Lease Summary Sheet (the "**Cash Security Deposit**"), which shall be held by Landlord in accordance with Section 7.5 below, or (ii) an irrevocable letter of credit to Landlord which shall (a) be in the amount specified in the Lease Summary Sheet and otherwise in the form attached hereto as **Exhibit 5**; (b) issued by a bank with a rating of A or better and otherwise reasonably acceptable to Landlord upon which presentment may be made in Boston, Massachusetts; and (c) be for a term of one (1) year, subject to extension in accordance with the terms hereof (the "**Letter of Credit**"). If Tenant delivers a Cash Security Deposit to Landlord, Tenant may later elect to substitute a Letter of Credit for the same on the terms and conditions contained in this Section 7. The Letter of Credit shall be held by Landlord, without liability for interest, as security for the faithful performance by Tenant of all of the terms, covenants and conditions of this Lease by the Tenant to be kept and performed during the Term. In no event shall the Letter of Credit be deemed to be a prepayment of Rent nor shall it be considered a measure of liquidated damages. Unless the Letter of Credit is automatically renewing, at least thirty (30) days prior to the maturity date of the Letter of Credit (or any replacement Letter of Credit), Tenant shall deliver to Landlord a replacement Letter of Credit which shall have a maturity date no earlier than the next anniversary of the Term Commencement Date or one (1) year from its date of delivery to Landlord, whichever is later.

**7.2 Application of Proceeds of Letter of Credit.** Upon an Event of Default, or if any proceeding shall be instituted by or against Tenant pursuant to any of the provisions of any Act of Congress or State law relating to bankruptcy, reorganizations, arrangements, compositions or other relief from creditors (and, in the case of any proceeding instituted against it, if Tenant shall fail to have such proceedings dismissed within sixty (60) days) or if Tenant is adjudged bankrupt or insolvent as a result of any such proceeding, Landlord at its sole option may draw down all or a part of the Letter of Credit. The balance of any Letter of Credit cash proceeds shall be held in accordance with Section 7.5 below. Should the entire Letter of Credit, or any portion thereof, be drawn down by Landlord, Tenant shall, upon the written demand of Landlord, deliver a replacement Letter of Credit in the amount drawn, and Tenant's failure to do so within ten (10) days after receipt of such written demand shall constitute an additional Event of Default hereunder. The application of all or any part of the cash proceeds of the Letter of Credit

to any obligation or default of Tenant under this Lease shall not deprive Landlord of any other rights or remedies Landlord may have nor shall such application by Landlord constitute a waiver by Landlord.

**7.3 Transfer of Letter of Credit.** In the event that Landlord transfers its interest in the Premises, Tenant shall upon notice from and at no material cost to Landlord, deliver to Landlord an amendment to the Letter of Credit or a replacement Letter of Credit naming Landlord's successor as the beneficiary thereof. If Tenant fails to deliver such amendment or replacement within ten (10) business days after written notice from Landlord, Landlord shall have the right to draw down the entire amount of the Letter of Credit and hold the proceeds thereof in accordance with Section 7.5 below.

**7.4 Credit of Issuer of Letter of Credit.** In event of a material adverse change in the financial position of any bank or institution which has issued the Letter of Credit or any replacement Letter of Credit hereunder, Landlord reserves the right to require that Tenant change the issuing bank or institution to another bank or institution reasonably approved by Landlord. Tenant shall, within ten (10) days after receipt of written notice from Landlord, which notice shall include the basis for Landlord's reasonable belief that there has been a material adverse change in the financial position of the issuer of the Letter of Credit, replace the then-outstanding letter of credit with a like Letter of Credit from another bank or institution approved by Landlord.

**7.5 Cash Proceeds of Letter of Credit.** Landlord shall hold the Cash Security Deposit and/or the balance of proceeds remaining after a draw on the Letter of Credit (each hereinafter referred to as the "**Security Deposit**") as security for Tenant's performance of all its Lease obligations. After an Event of Default, Landlord may apply the Security Deposit, or any part thereof, to Landlord's damages without prejudice to any other Landlord remedy. Landlord shall keep the Security Deposit in an interest bearing account and any such interest shall be payable to Tenant. Landlord may co-mingle the Security Deposit with Landlord's funds. If Landlord conveys its interest under this Lease, the Security Deposit, or any part not applied previously, may be turned over to the grantee in which case Tenant shall look solely to the grantee for the proper application and return of the Security Deposit. So long as the Property is subject to a Mortgage, Landlord shall cause its first Mortgagee to hold any Cash Security Deposit, subject to the terms of this Section 7.

**7.6 Return of Security Deposit or Letter of Credit.** Should Tenant comply with all of such terms, covenants and conditions and promptly pay all sums payable by Tenant to Landlord hereunder, the Security Deposit and/or Letter of Credit or the remaining proceeds therefrom, as applicable, shall be returned to Tenant within forty-five (45) days after the end of the Term, less any portion thereof which may have been utilized by Landlord to cure any default or applied to any actual damage suffered by Landlord.

**8. NO SECURITY INTEREST IN TENANT'S PROPERTY.** Landlord hereby waives any right to claim a security interest or lien right in Tenant's Property (hereinafter defined). Landlord hereby agrees to execute a waiver and agreement in Landlord's commercially reasonable form for the benefit of any national banking association or institutional lender of Tenant (provided, however, that it is understood and agreed by Tenant that the foregoing provisions shall not affect the prohibition set forth in Section 26.13 hereof).

## **9. UTILITIES, LANDLORD'S SERVICES**

**9.1 Electricity.** Commencing on the Term Commencement Date, Tenant shall pay all charges for electricity furnished to the Premises and any equipment exclusively serving the Premises, as additional rent, based on separate meters installed as part of the Landlord Work. Tenant shall, at Tenant's sole cost and expense, maintain and keep in good order, condition and repair the metering equipment used to measure electricity furnished to the Premises and any equipment exclusively serving the same. Tenant shall pay the full amount of any charges attributable to such meter on or before the due date therefor

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directly to the supplier thereof.

**9.2 Water.** Tenant shall pay all charges for water furnished to the Premises and/or any equipment exclusively serving the Premises as additional rent, based on separate checkmeters installed as part of the Landlord Work. Tenant shall, at Tenant's sole cost and expense, maintain and keep in good order, condition and repair the metering equipment used to measure water furnished to the Premises and any equipment exclusively serving the same. Tenant shall pay the full amount of any charges attributable to such meter on or before the due date therefor either to Landlord based on Landlord's reading of such checkmeter or directly to the supplier thereof, in either case without mark-up by Landlord.

**9.3 Gas.** Tenant shall pay all charges for gas furnished to the Premises and/or any equipment exclusively serving the Premises as additional rent, based on separate meters installed as part of the Landlord Work. Tenant shall, at Tenant's sole cost and expense, maintain and keep in good order, condition and repair the metering equipment used to measure gas furnished to the Premises and any equipment exclusively serving the same. Tenant shall pay the full amount of any charges attributable to such meter on or before the due date therefor directly to the supplier thereof.

**9.4 Other Utilities.** Subject to Landlord's reasonable Rules and Regulations governing the same, Tenant shall obtain and pay, as and when due, for all other utilities and services consumed in and/or furnished to the Premises, together with all taxes, penalties, surcharges and maintenance charges pertaining thereto.

### **9.5 Interruption or Curtailment of Utilities.**

(a) When necessary by reason of accident or emergency, or for repairs, alterations, replacements or improvements which in the reasonable judgment of Landlord are desirable or necessary to be made, Landlord reserves the right, upon as much prior notice to Tenant as is practicable under the circumstances and no less than three business days' notice except in the event of an emergency, to interrupt, curtail, or stop (i) the furnishing of hot and/or cold water, and (ii) the operation of the plumbing and electric systems (Landlord agreeing to use commercially reasonable efforts to (A) minimize the duration of any such interruption and (B) schedule such interruptions that are planned by Landlord, if any, after Tenant's normal business hours; if such duration is reasonably expected to be for greater than 24 hours during any of Tenant's normal business hours (as defined in Section 2.4(a) above), then Landlord shall provide alternate services during such period). Landlord shall exercise reasonable diligence to eliminate the cause of any such interruption, curtailment, stoppage or suspension, but there shall be no diminution or abatement of Rent or other compensation due from Landlord to Tenant hereunder, nor shall this Lease be affected or any of Tenant's obligations hereunder reduced, and Landlord shall have no responsibility or liability for any such interruption, curtailment, stoppage, or suspension of services or systems.

(b) Notwithstanding anything to the contrary in this Lease contained, if the Premises are rendered untenable, in whole or in part, due to the failure of Landlord to (i) provide any of Landlord's Services, or (ii) Landlord's failure to provide reasonable means of access or egress to/from the Premises or to perform its repair or maintenance obligations such that, in either event, for the duration of the Interruption Cure Period (hereinafter defined), the continued operation in the ordinary course of Tenant's business in any portion of the Premises (the "**Affected Portion**") is materially and adversely affected and if Tenant ceases to use the Affected Portion in the ordinary course as the direct result of such lack of service or failure, then, provided that Landlord's inability to cure such condition is not caused by the negligence or willful misconduct of any of the Tenant Parties, Base Rent, and additional rent on account of Operating Costs and Taxes shall be abated in proportion to such untenability until the day such condition is completely corrected commencing on the first (1<sup>st</sup>) day of such lack of service or the date on which Landlord received notice of such failure, as the case may be. For purposes hereof, the

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**"Interruption Cure Period"** shall be defined as (A) three (3) consecutive business days after Landlord's receipt of written notice from Tenant of the condition causing the lack of service in the Affected Portion or lack of reasonable means of access/egress to/from the Premises, or (B) ten (10) consecutive business days after receipt of written notice from Tenant of the Landlord default in its repair and maintenance obligations (which written notice shall indicate that such default is causing such material and adverse effects on Tenant's business operations in the ordinary course) (such notice by Tenant to Landlord under subsection (A) or (B), the "**Interruption Notice**"). If the Interruption Notice indicates that the interruption event giving rise to a rent abatement pursuant to this paragraph materially and adversely interferes with Tenant's use of at least 50% of the Premises (as measured in rentable square feet) or a portion of the Premises necessary to conduct Tenant's research and development or light manufacturing uses substantially in the ordinary course, and, despite Tenant's good faith efforts to take reasonable prudent measures and safeguards to avoid such interference and/or effects, such event materially and adversely interferes with Tenant's use of at least 50% of the Premises (as measured in rentable square feet) or portion of the Premises necessary to conduct Tenant's research and development or light manufacturing uses substantially in the ordinary course for a period of at least three hundred sixty-five (365) consecutive days after the Interruption Notice, then provided that Landlord's inability to cure such condition is not caused by the negligence or willful misconduct of any of the Tenant Parties, Tenant may elect to terminate this Lease upon at least thirty (30) days' written notice to Landlord; provided, however, if such interruption event ceases within such 30 day period, then Tenant's termination notice shall be null and void. The provisions of this Section 9.5(b) shall not apply in the event of Casualty or Taking (which shall be governed by Section 15 below).

**9.6 Landlord's Services.** Subject to reimbursement to the extent provided pursuant to Section 5.2 above, Landlord shall provide the services described in Exhibit 6 attached hereto and made a part hereof ("**Landlord's Services**").

## **10. MAINTENANCE AND REPAIRS**

**10.1 Maintenance and Repairs by Tenant.** Tenant shall keep neat and clean and free of insects, rodents, vermin and other pests and in good repair, order and condition the Premises, including without limitation the entire interior of the Premises, all electronic, phone and data cabling and related equipment that is installed by or for the exclusive benefit of the Tenant (whether located in the Premises or other portions of the Building), all fixtures, equipment and lighting therein, electrical equipment wiring, doors, non-structural walls, and floor coverings, reasonable wear and tear and damage by Casualty excepted. Tenant shall be solely responsible, at Tenant's sole cost and expense, for the proper maintenance of all building systems, life-safety, sanitary, electrical, heating, air conditioning, plumbing, security or other systems and of all equipment and appliances to the extent exclusively serving the Premises. Tenant agrees to provide regular maintenance by contract with a reputable qualified service contractor for the heating and air conditioning equipment servicing the Premises. Such maintenance contract and contractor shall be subject to Landlord's reasonable approval. Tenant, at Landlord's request, shall at reasonable intervals provide Landlord with copies of such contracts and maintenance and repair records and/or reports.

**10.2 Maintenance and Repairs by Landlord.** Except as otherwise provided in Section 15, and subject to Tenant's obligations in Section 10.1 above, Landlord shall maintain and keep in good condition, consistent with first class office and laboratory buildings, the components of the Building constructed by Landlord or its predecessors-in-interest, including the Landlord's Work, Building foundation, the roof and roof systems, Building structure, exterior wall system, façade, weight bearing walls, windows, structural floor and ceiling slabs and columns and the common components of life-safety, sanitary, electrical, heating, air conditioning, plumbing, security, mechanical and other Building systems. In addition, Landlord shall operate and maintain the Common Areas in substantially the same manner as comparable first class buildings in the vicinity of the Premises.

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**10.3 Accidents to Sanitary and Other Systems.** Tenant shall give to Landlord prompt notice of any fire or accident in the Premises or in the Building and of any damage to, or defective condition in, any part or appurtenance of the Building including, without limitation, sanitary, electrical, ventilation, heating and air conditioning or other systems located in, or passing through, the Premises (provided that it shall not be a default of Tenant if it fails to do so). Except as otherwise provided in Section 15, and subject to Tenant's obligations in Section 10.1 above, such damage or defective condition shall be remedied by Landlord with reasonable diligence, but, subject to Section 14.5 below, if such damage or defective condition was caused by any of the Tenant Parties, the cost to remedy the same shall be paid by Tenant.

**10.4 Floor Load—Heavy Equipment.** Tenant shall not place a load upon any floor of the Premises exceeding the floor load per square foot of area which such floor was designed to carry (i.e. 150 pounds per square foot) and which is allowed by Legal Requirements. Landlord reserves the right to prescribe the weight and position of all safes, heavy machinery, heavy equipment, freight, unusually bulky matter or fixtures (collectively, "**Heavy Equipment**"), which shall be placed so as to distribute the weight. Heavy Equipment shall be placed and maintained by Tenant at Tenant's expense in settings sufficient in Landlord's reasonable judgment to absorb and prevent vibration, noise and annoyance. Tenant shall not move any Heavy Equipment into or out of the Building without giving Landlord prior written notice thereof and observing all of Landlord's Rules and Regulations with respect to the same. If such Heavy Equipment requires special handling, Tenant agrees to employ only persons holding a Master Rigger's License to do said work, and that all work in connection therewith shall comply with Legal Requirements. Any such moving shall be at the sole risk and hazard of Tenant and Tenant will defend, indemnify and save Landlord and Landlord's agents (including without limitation its property manager), contractors and employees (collectively with Landlord, the "**Landlord Parties**") harmless from and against any and all claims, damages, losses, penalties, costs, expenses and fees (including without limitation reasonable legal fees) (collectively, "**Claims**") resulting directly or indirectly from such moving, except to the extent resulting from Landlord's negligence or willful misconduct. Proper placement of all Heavy Equipment in the Premises shall be Tenant's responsibility.

## **11. ALTERATIONS AND IMPROVEMENTS BY TENANT**

**11.1 Landlord's Consent Required.** Tenant shall not make any alterations, decorations, installations, removals, additions or improvements (collectively with Tenant's Work, "**Alterations**") in or to the Premises without Landlord's prior written approval of the contractor(s), written plans and specifications and a time schedule therefor, which approval of contractor(s) and schedule shall not be unreasonably withheld, conditioned or delayed (and shall not require Tenant to conduct such Alterations after normal business hours or on weekends except as otherwise expressly required pursuant to this Lease or as may be required pursuant to Section 11.3 below). Notwithstanding anything to the contrary, in no event shall any Alterations involve the removal of any improvements made by, or paid for by, Landlord without Landlord's prior approval in Landlord's sole discretion. Landlord shall respond to any request for approval of Alterations within ten (10) Business Days after receipt of the foregoing required information and shall promptly notify Tenant if any submission is incomplete. Notwithstanding the foregoing, Landlord's consent shall not be required with respect to any Alterations that are purely decorative in nature nor with respect to non-structural Alterations costing less than \$250,000 in any one instance (and \$750,000 in the aggregate per year) so long as such Alterations do not materially adversely affect the roof, Building systems or Building exterior (each, a "**Permitted Alteration**"), provided Tenant shall provide Landlord with reasonably detailed written notice thereof. Landlord reserves the right to require that Tenant use Landlord's preferred vendor(s) for any Alterations that involve roof penetrations, alarm tie-ins, sprinklers, fire alarm and other life safety equipment, provided that such vendors are available at commercially reasonable rates. Tenant shall not make any amendments or additions to plans and specifications (other than minor amendments in the nature of field changes) approved by Landlord without Landlord's prior written consent (the standard of which consent shall be governed by the provisions of this Section 11). Landlord's approval of Alterations shall not be unreasonably withheld,

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conditioned or delayed. Notwithstanding the foregoing, Landlord may withhold its consent in its sole discretion (a) to any Alteration to or affecting the roof (other than the Rooftop Premises) and/or materially and adversely affecting any building systems, (b) with respect to matters of aesthetics relating to Alterations to or affecting the exterior of the Building, and (c) to any Alteration adversely affecting the Building structure, with Tenant being obligated to provide Landlord with reasonable evidence that such Alteration does not adversely affect any portion of the Building structure. Tenant shall be responsible for all elements of the design of Tenant's plans (including, without limitation, compliance with Legal Requirements, functionality of design, the structural integrity of the design, the configuration of the Premises and the placement of Tenant's furniture, appliances and equipment), and Landlord's approval of Tenant's plans shall in no event relieve Tenant of the responsibility for such design. Landlord shall provide Tenant with copies of Landlord Work Plans and all other plans for the Building in Landlord's possession or control. Landlord shall have no liability or responsibility for any claim, injury or damage alleged to have been caused by the particular materials (whether building standard or non-building standard), appliances or equipment selected by Tenant in connection with any work performed by or on behalf of Tenant. Except as otherwise expressly set forth herein, all Alterations shall be done at Tenant's sole cost and expense and at such times and in such manner as Landlord may from time to time reasonably designate (provided that Tenant shall not be required to conduct such Alterations after normal business hours or on weekends except as otherwise expressly required pursuant to Section 11.2 or as may be required pursuant to Section 11.3). If Tenant shall make any Alterations (other than Tenant's Work, except to the extent set forth in the immediately following sentence) that are specialized Alterations inconsistent with first class office and laboratory improvements then-customarily found in similar buildings in the Town of Lexington, Massachusetts ("**Specialty Alterations**") (and expressly excluding any non-structural Alterations to the office areas of the Premises and any matters identified as "Tenant" in the Matrix), then Landlord may

elect to require Tenant at the expiration or sooner termination of the Term to remove such Specialty Alterations and repair any damage to the Premises caused by such removal (which election shall be made at the time of Landlord's approval of such Alterations). In addition, Tenant acknowledges that Landlord has the right to designate any Specialty Alterations associated with Tenant's light manufacturing operations in the Premises for removal at the time that Landlord approves Tenant's plans for Tenant's Work (but Landlord shall have no right to require the removal of any other portion of Tenant's Work to the extent the Final Construction Drawings are consistent with Exhibit 3C attached hereto). Tenant shall provide Landlord with reproducible record drawings (in CAD format) of all Alterations susceptible to the creation of record drawings within sixty (60) days after completion thereof. During the Term only, Tenant shall be treated as the owner of all Alterations for all purposes under this Lease, including but not limited to the depreciation of such Alterations.

**11.2 After-Hours.** Landlord and Tenant recognize that to the extent Tenant elects to perform some or all of the Alterations during times other than normal construction hours (i.e., Monday-Friday, 7:00 a.m. to 6:00 p.m., excluding holidays), Landlord may need to make arrangements to have supervisory personnel on site. Accordingly, Landlord and Tenant agree as follows: Tenant shall give Landlord at least two (2) business days' prior written notice of any time outside of normal construction hours when Tenant intends to perform any Alterations (the "**After-Hours Work**"). In addition, if construction during normal construction hours unreasonably disturbs other tenants of the Building, in Landlord's sole discretion, Landlord may require Tenant to stop the performance of Alterations during normal construction hours and to perform the same after hours. From and after the Execution Date, Landlord shall include substantially similar provisions in any other leases at the Building and shall not enforce such provisions in a discriminatory manner.

**11.3 Harmonious Relations.** Tenant agrees that it will not, either directly or indirectly, use any contractors and/or materials if their use creates, or is reasonably expected to create, any difficulty, whether in the nature of a labor dispute or otherwise, with other contractors and/or labor engaged by Tenant or Landlord or others in the construction, maintenance and/or operation of the Building, the Property or any part thereof. In the event of any such difficulty, upon Landlord's request, Tenant shall cause all contractors, mechanics or laborers causing such difficulty to leave the Property

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immediately. From and after the Execution Date, Landlord shall include substantially similar provisions in any other leases at the Building and shall not enforce such provisions in a discriminatory manner.

**11.4 Liens.** No Alterations shall be undertaken by Tenant until (i) Tenant obtains statutory waivers of liens from all contractors for such Alteration; and (ii) with respect to work (excluding Tenant's Work performed by, through or under The Richmond Group, Inc.) in excess of \$600,000 in the aggregate, Tenant has procured appropriate surety payment and performance bonds which shall name Landlord as an additional obligee and has filed lien bond(s) (in jurisdictions where available) on behalf of such contractors. Any mechanic's lien filed against the Premises or the Building for work claimed to have been done for, or materials claimed to have been furnished to, Tenant shall be discharged by Tenant within fifteen (15) days after notice thereof, at Tenant's expense by filing the bond required by law or otherwise.

**11.5 General Requirements.** Unless Landlord and Tenant otherwise agree in writing, Tenant shall (a) procure or cause others to procure on its behalf all necessary permits before undertaking any Alterations in the Premises (and provide copies thereof to Landlord); (b) perform all of such Alterations in a good and workmanlike manner, employing materials of good quality and in compliance with Landlord's construction Rules and Regulations, all insurance requirements of this Lease and Legal Requirements; and (c) defend, indemnify and hold the Landlord Parties harmless from and against any and all Claims occasioned by or growing out of the performance of such Alterations. Tenant shall cause contractors employed by Tenant to (i) carry Worker's Compensation Insurance in accordance with statutory requirements, (ii) carry Automobile Liability Insurance and Commercial General Liability Insurance (A) naming Landlord as an additional insured, and (B) covering such contractors on or about the Premises in the amounts stated in Section 14 hereof or in such other reasonable amounts as Landlord shall require, and (iii) cause such contractors submit binders or other evidence of insurance evidencing such coverage to Landlord prior to the commencement of any such Alterations.

## 12. SIGNAGE

**12.1 Restrictions.** Tenant shall have the right to install Building standard signage identifying Tenant's business at the entrance to the Premises, which signage shall be subject to Landlord's prior written consent (which consent shall not be unreasonably withheld, conditioned or delayed so long as it complies with the signage guidelines attached hereto as Exhibit 11 (the "**Signage Guidelines**")). Subject to the foregoing, and subject to Section 12.2 below, Tenant shall not place or suffer to be placed or maintained on the exterior of the Premises, or any part of the interior visible from the exterior thereof, any sign, banner, advertising matter or any other thing of any kind (including, without limitation, any hand-lettered advertising), and shall not place or maintain any decoration, letter or advertising matter on the glass of any window or door of the Premises visible from the exterior of the Premises without first obtaining Landlord's written approval. No signs may be put on or in any window or elsewhere if visible from the exterior of the Building. No blinds may be put on or in any window or elsewhere if visible from the exterior of the Building without Landlord's reasonable prior consent.

**12.2 Exterior Signage.** Provided that and for so long as Tenant meets the Occupancy Threshold, Tenant shall have the right to erect and maintain one (1) sign on the exterior of the Building at the entrance to the Premises ( the "**Exterior Signage**"), provided (i) the Exterior Signage complies with the Signage Guidelines and all Legal Requirements (and Tenant shall have obtained any necessary permits prior to erecting the Exterior Signage), (ii) the location of the Exterior Signage shall be subject to Landlord's reasonable approval, (iii) the materials, design, lighting and method of installation of the Exterior Signage, and any requested changes thereto, shall be subject to Landlord's prior written approval, which approval shall not be unreasonably withheld, conditioned or delayed, and (iv) Tenant shall at all times maintain the Exterior Signage in good order, condition and repair and shall remove the Exterior Signage at the expiration or earlier termination of the Term hereof or upon Landlord's written demand after the failure of Tenant to comply with the provisions of this Section 12.2, and shall repair any

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damage to the Building caused by the Exterior Signage or the installation or removal thereof. Tenant shall have the right, from time to time throughout the term of this Lease, to replace its signage (if any) with signage which is equivalent to the signage being replaced, subject to all of the terms and conditions of this Section 12.2. Landlord shall list Tenant on any multi-tenant Building monument signage maintained by Landlord in a manner comparable to those listings provided to other tenants in the Building. In no event shall Landlord disapprove of the inclusion of Tenant corporate branding and logo (which may include the use of Tenant's name) on any of Tenant's signage. Neither Landlord nor any of its affiliates shall make or permit any improvements to the Property that would adversely affect the visibility of signage installed by Tenant pursuant to this Section 12.2 from Hartwell Avenue.

## 13. ASSIGNMENT, MORTGAGING AND SUBLETTING

**13.1 Landlord's Consent Required.** Except as expressly otherwise set forth herein, Tenant shall not, without Landlord's prior written consent, assign, sublet, mortgage, license, transfer or encumber this Lease or the Premises in whole or in part whether by changes in the control of Tenant (other than as the result of transfers of interests among parties holding a direct or indirect interest in Tenant), or permit the occupancy of all or any portion of the Premises by any person or entity other than Tenant's employees (each of the foregoing, a "**Transfer**"). Any purported Transfer made without Landlord's consent, if required hereunder, shall be void and confer no rights upon any third person, provided that if there is a Transfer, Landlord may collect rent from the transferee without waiving the prohibition against Transfers, accepting the transferee, or releasing Tenant from full performance under this Lease. No Transfer shall relieve Tenant of its primary obligation as party-Tenant hereunder, nor shall it reduce or increase Landlord's obligations under this Lease. In no event shall the issuance of any beneficial interests in Tenant or any entity holding an interest in Tenant or the trading of any stock in any entity directly or indirectly holding an interest in Tenant on a nationally-recognized stock exchange be deemed to result in a Transfer hereunder.

### 13.2 Landlord's Recapture Right

(a) Tenant shall, prior to entering into any Transfer (other than a Permitted Transfer (hereinafter defined)) for all or substantially all of the balance of the Term of this Lease and affecting fifty percent (50%) or more of the Premises (individually or in the aggregate with other Transfers (other than a Permitted Transfer) in effect) (such a Transfer, a "**Material Transfer**"), give a written notice (the "**Recapture Notice**") to Landlord which: (i) states that Tenant desires to make a Material Transfer, (ii) identifies the affected portion of the Premises (the "**Recapture Premises**"), (iii) identifies the date on which the Material Transfer is proposed to take effect, and (iv) offers to Landlord to terminate this Lease with respect to the Recapture Premises. Landlord shall have ten (10) business days within which to respond to the Recapture Notice and, if Landlord accepts Tenant's Recapture Notice, then this Lease shall terminate with respect to the Recapture Premises effective on the proposed effective date of such Material Transfer as set forth in the Recapture Notice.

(b) If Tenant does not enter into a Transfer on the terms and conditions contained in the Recapture Notice on or before the date which is one hundred eighty (180) days after the earlier of: (x) the expiration of the 10-business day period specified in Section 13.2(a) above, or (y) the date that Landlord notifies Tenant that Landlord will not accept Tenant's offer contained in the Recapture Notice, *time being of the essence*, then prior to entering into any Transfer after such 180-day period, Tenant must deliver to Landlord a new Recapture Notice in accordance with Section 13.2(a) above

(c) Notwithstanding anything to the contrary contained herein, if Landlord notifies Tenant that it accepts the offer contained in the Recapture Notice or any subsequent Recapture Notice, Tenant shall have the right, for a period of ten (10) days following receipt of such notice from Landlord, *time being of the essence*, to notify Landlord in writing that it wishes to withdraw such offer and this Lease shall continue in full force and effect.

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**13.3 Standard of Consent to Transfer.** If Landlord does not timely give written notice to Tenant accepting a Recapture Offer or declines to accept the same, then Landlord agrees that, subject to the provisions of this Section 13, Landlord shall not unreasonably withhold, condition or delay its consent to a Transfer on the terms contained in the Recapture Notice to an entity which will use the Premises for the Permitted Uses and: (a) with respect to any assignment of this Lease or a sublet of all or substantially all of the Premises, has a tangible net worth and other financial indicators at least equal to Tenant's as of the date of the Recapture Notice; and (b) has a business reputation compatible with the operation of a first-class combination laboratory, research, development and office building and in any event, shall grant or deny such consent within fifteen (15) business days after Tenant's request for such consent.

**13.4 Listing Confers no Rights.** The listing of any name other than that of Tenant, whether on the doors of the Premises or on the Building directory, or otherwise, shall not operate to vest in any such other person, firm or corporation any right or interest in this Lease or in the Premises or be deemed to effect or evidence any consent of Landlord, it being expressly understood that any such listing is (except to the extent of Tenant's express signage rights provided under Section 12) a privilege extended by Landlord revocable at will by written notice to Tenant.

**13.5 Profits In Connection with Transfers.** Tenant shall, within thirty (30) days of receipt thereof, pay to Landlord fifty percent (50%) of any rent, sum or other consideration to be paid or given in connection with any Transfer, either initially or over time, after deducting reasonable actual out-of-pocket legal and brokerage expenses incurred by Tenant, rent concessions, tenant improvement allowances, and other third party costs incurred by Tenant in connection with such Transfer and the depreciated value (in accordance with generally accepted accounting principles) of the cost of Alterations performed at Tenant's expense to prepare the Premises for Tenant's use and occupancy (i.e amounts in excess of Landlord's Contribution), prorated according to the fraction of rentable area of the Premises affected the Transfer, in excess of Rent hereunder as if such amount were originally called for by the terms of this Lease as additional rent.

**13.6 Prohibited Transfers.** Notwithstanding any contrary provision of this Lease, Tenant shall have no right to make a Transfer unless on both (i) the date on which Tenant notifies Landlord of its intention to enter into a Transfer and (ii) the date on which such Transfer is to take effect, Tenant is not in default of any of its obligations under this Lease (it being understood and agreed that if Tenant cures any default(s) within the applicable cure period(s) provided in Section 20 below, then Tenant shall thereafter be entitled to make the Transfer in question). Notwithstanding anything to the contrary contained herein, Tenant agrees that in no event shall Tenant make a Transfer to (a) any government agency so long as no other portion of the Building is leased to a governmental agency; (b) any tenant, subtenant or occupant of other space in the Building provided that comparable space is then available for lease in the Building; or (c) any entity with whom Landlord shall have negotiated for space in the Property in the three (3) months immediately preceding such proposed Transfer.

### 13.7 Permitted Transfers.

(a) Notwithstanding anything to the contrary herein contained, Tenant shall have the right, without obtaining Landlord's consent, to (i) make a Transfer to an Affiliate, and (ii) to assign all of its right, title and interest in and to the Premises pursuant to this Lease to a Successor, provided that, in the event of an assignment of Tenant's interest in this Lease (except as the result of a merger), such Affiliate or Successor, as the case may be, and Tenant execute and deliver to Landlord a commercially reasonable assignment and assumption agreement whereby such Affiliate or Successor, as the case may be, shall agree to be independently bound by and upon all of the covenants, agreements, terms, provisions and conditions set forth in this Lease on the part of Tenant to be performed, and whereby such Affiliate or Successor, as the case may be, shall expressly agree that the provisions of this Section 13 shall, notwithstanding such assignment, continue to be binding upon it with respect to all future Transfers.

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Tenant shall deliver such assignment and assumption agreement to Landlord prior to the effective date of such assignment unless Tenant is contractually or legally prohibited from doing so, in which event such agreement shall be delivered to Landlord within ten (10) days after the effective date thereof. For the purposes hereof, an "**Affiliate**" shall be defined as any entity which is controlled by, is under common control with, or which controls Tenant. For the purposes hereof, a "**Successor**" shall be defined as any entity into or with which Tenant is merged or with which Tenant is consolidated or which acquires all or substantially all of

Tenant's stock or assets, or any other corporate reorganization of Tenant, provided that the surviving entity shall have a net worth and other financial indicators at least equal to Tenant's immediately prior to such event.

(b) Notwithstanding any provision to the contrary in this Lease, occupancy of less than five thousand (5,000) rentable square feet of the Premises by companies, firms or other entities (i) who are members of a group with whom Tenant has a contractual or other relationship providing for cooperative or collaborative research or development work, who are or typically would be located by Tenant in one of its facilities, and/or (ii) in which Tenant has a beneficial interest and which are actively engaged in research activities using technology, techniques and/or equipment developed by Tenant, shall not be a Transfer for the purposes of this Section 13 and shall be permitted without the necessity of obtaining Landlord's consent thereto, but Tenant shall provide Landlord with prior written notice thereof (which notice shall include the identity of such entities, the number of square feet in occupancy by such entities and such other information reasonably required for financing, insurance and other risk management purposes, but which notice may otherwise be limited in detail to the extent required by applicable confidentiality agreements).

(c) Notwithstanding any provision to the contrary in this Lease, occupancy of less than five thousand (5,000) rentable square feet of the Premises by companies, firms or other entities who are Tenant's vendors, customers and/or suppliers, who are or typically would be located by Tenant in one of its facilities shall not be a Transfer for the purposes of this Section 13 and shall be permitted without the necessity of obtaining Landlord's consent thereto, but Tenant shall provide Landlord with prior written notice thereof (which notice shall include the identity of such entities, the number of square feet in occupancy by such entities and such other information reasonably required for financing, insurance and other risk management purposes, but which notice may otherwise be limited in detail to the extent required by applicable confidentiality agreements).

(d) Notwithstanding the provisions of this Section 13.7, no transaction or series of transactions which are effected solely for the purpose of qualifying as a transaction which does not require Landlord's consent (i.e. and thereby avoiding the operation of the provisions of this Section 13) shall be permitted pursuant to this Section 13.7.

(e) Transfers not requiring Landlord consent pursuant to this Section 13.7 are referred to herein as "**Permitted Transfers.**"

## **14. INSURANCE; INDEMNIFICATION; EXCULPATION**

### **14.1 Tenant's Insurance.**

(a) Tenant shall procure, pay for and keep in force throughout the Term (and for so long thereafter as Tenant remains in occupancy of the Premises) commercial general liability insurance insuring Tenant on an occurrence basis against all claims and demands for personal injury liability (including, without limitation, bodily injury, sickness, disease, and death) or damage to property which may be claimed to have occurred from and after the time any of the Tenant Parties shall first enter the Premises, of not less than Two Million Dollars (\$2,000,000) per occurrence, Three Million (\$3,000,000) in the aggregate, and from time to time thereafter shall be not less than such higher amounts, if procurable, as may be reasonably required by Landlord if consistent with limits carried by similar tenants

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at similar properties in the vicinity (but in any event such amounts shall not be increased more often than once every five years commencing at the conclusion of the fifth (5<sup>th</sup>) Rent Year). Tenant shall also carry umbrella liability coverage in an amount of no less than Five Million Dollars (\$5,000,000). Such policy shall also include contractual liability coverage covering Tenant's liability assumed under this Lease, including without limitation Tenant's indemnification obligations under Section 14.2 below. Such insurance policy(ies) shall name Landlord, Landlord's managing agent and such persons claiming by, through or under them identified by Landlord in writing, if any, as additional insureds.

(b) Tenant shall take out and maintain throughout the Term a policy of fire, vandalism, malicious mischief, extended coverage and so-called "all risk" coverage insurance in an amount equal to one hundred percent (100%) of the replacement cost insuring (i) all items or components of Alterations (collectively, the "**Tenant-Insured Improvements**"), and (ii) all of Tenant's furniture, equipment, fixtures and property of every kind, nature and description related or arising out of Tenant's leasehold estate hereunder, which may be in or upon the Premises or the Building, including without limitation Tenant's Rooftop Equipment (collectively, "**Tenant's Property**"). Such insurance shall insure the interests of both Landlord and Tenant as their respective interests may appear from time to time.

(c) Tenant shall take out and maintain a policy of business interruption insurance throughout the Term sufficient to cover at least twelve (12) months of Rent due hereunder and Tenant's business losses during such twelve (12) month period.

(d) Tenant shall procure and maintain at its sole expense such additional insurance as may be necessary to comply with any Legal Requirements.

(e) The insurance required pursuant to Sections 14.1(a), (b), (c) and (d) (collectively, "**Tenant's Insurance Policies**") shall be effected with insurers reasonably approved by Landlord, with a rating of not less than "A-/VII" in the current *Best's Insurance Reports*, and authorized to do business in the Commonwealth of Massachusetts under valid and enforceable policies. Tenant's Insurance Policies shall not be canceled or modified by Tenant without at least thirty (30) days' prior written notice, ten (10) days for nonpayment of premium, to each insured named therein. Tenant's Insurance Policies may include deductibles in an amount no greater than the greater of \$25,000 or commercially reasonable amounts. On or before the date on which any of the Tenant Parties shall first enter the Premises and thereafter prior to the expiration of each policy, Tenant shall deliver to Landlord certificates evidencing the coverage required hereunder with evidence satisfactory to Landlord of the payment of all premiums for such policies. In the event of any claim, and upon Landlord's request from time to time, Tenant shall deliver to Landlord complete copies of Tenant's Insurance Policies. Upon request of Landlord, Tenant shall deliver to any Mortgagee copies of the foregoing documents.

### **14.2 Indemnification.**

(a) Except to the extent caused by the negligence or willful misconduct of any of the Landlord Parties, Tenant shall defend, indemnify and save the Landlord Parties harmless from and against any and all Claims asserted by or on behalf of any person, firm, corporation or public authority arising from:

(i) Tenant's breach of any covenant or obligation under this Lease;

(ii) Any injury to or death of any person, or loss of or damage to property, sustained or occurring in, upon or at the Premises; and



(iii) Any injury to or death of any person, or loss of or damage to property (A) arising out of the use or occupancy of the Premises by any of the Tenant Parties, and (B) at the Property arising out the negligence or willful misconduct of any of the Tenant Parties.

(b) Except to the extent caused by the negligence or willful misconduct of any of the Tenant Parties, Landlord shall defend, indemnify and save the Tenant Parties harmless from and against any and all Claims asserted by or on behalf of any person, firm, corporation or public authority arising from:

(i) Landlord's breach of any covenant or obligation under this Lease; and

(ii) Any injury to or death of any person, or loss of or damage to property at the Property arising out the negligence or willful misconduct of any of the Landlord Parties.

**14.3 Property of Tenant.** Tenant covenants and agrees that, to the maximum extent permitted by Legal Requirements, all of Tenant's Property at the Premises shall be at the sole risk and hazard of Tenant, and that if the whole or any part thereof shall be damaged, destroyed, stolen or removed from any cause or reason whatsoever, no part of said damage or loss shall be charged to, or borne by, Landlord, except, subject to Section 14.5 hereof, to the extent such damage or loss is due to the negligence or willful misconduct of any of the Landlord Parties.

**14.4 Limitation of Landlord's Liability for Damage or Injury.** Landlord shall not be liable for any injury or damage to persons or property resulting from fire, explosion, falling plaster, steam, gas, air contaminants or emissions, electricity, electrical or electronic emanations or disturbance, water, rain or snow or leaks from any part of the Building or from the pipes, appliances, equipment or plumbing works or from the roof, street or sub-surface or from any other place or caused by dampness, vandalism, malicious mischief or by any other cause of whatever nature, except to the extent caused by or due to the negligence or willful misconduct of any of the Landlord Parties or Landlord's breach of this Lease and then, where notice and an opportunity to cure are appropriate (i.e., where Tenant has active and actual knowledge of such condition sufficiently in advance of the occurrence of any such injury or damage resulting therefrom as would have enabled Landlord to prevent such damage or loss had Tenant notified Landlord of such condition) only after (i) notice to Landlord of the condition claimed to have constituted negligence or willful misconduct, and (ii) the expiration of a reasonable time after notice has been received by Landlord without Landlord having commenced to take all reasonable and practicable means to cure or correct such condition; and pending such cure or correction by Landlord, Tenant shall take all reasonably prudent temporary measures and safeguards to prevent any injury, loss or damage to persons or property. Notwithstanding the foregoing, in no event shall any of the Landlord Parties be liable for any property loss which is covered by insurance policies actually carried or required to be carried by this Lease; nor shall any of the Landlord Parties be liable for any such damage caused by other tenants or persons in the Building or caused by operations in construction of any private, public or quasi-public work. Nothing in this Section 14.4 shall derogate or diminish Landlord's obligations under Section 10.2 above.

**14.5 Waiver of Subrogation; Mutual Release.** Landlord and Tenant each hereby waives on behalf of itself and its property insurers (none of which shall ever be assigned any such claim or be entitled thereto due to subrogation or otherwise) any and all rights of recovery, claim, action, or cause of action against the other and its agents, officers, servants, partners, shareholders, or employees (collectively, the "**Related Parties**") for any loss or damage that may occur to or within the Premises or the Building or any improvements thereto, or any personal property of such party therein which is insured against under any property insurance policy actually being maintained by the waiving party from time to time, even if not required hereunder, or which would be insured against under the terms of any insurance policy required to be carried or maintained by the waiving party hereunder, whether or not such insurance coverage is actually being maintained, including, in every instance, such loss or damage that may be caused by the negligence of the other party hereto and/or its Related Parties. Landlord and Tenant each agrees to cause appropriate clauses to be included in its property insurance policies necessary to implement the foregoing provisions.

**14.6 Tenant's Acts—Effect on Insurance.** Tenant shall not do or permit any Tenant Party to do any act or thing upon the Premises or elsewhere in the Building which will invalidate or be in conflict with any insurance policies covering the Building and the fixtures and property therein of which Tenant has prior written notice (Landlord acknowledging that the use of the Premises for the Permitted Uses, generally, is not in violation of the provisions of this sentence). If by reason of the failure of Tenant to comply with the provisions hereof the insurance rate applicable to any policy of insurance shall at any time thereafter be higher than it otherwise would be, Tenant shall cease the applicable acts or reimburse Landlord upon demand for that part of any insurance premiums which shall have been charged because of such failure by Tenant, together with interest at the Default Rate until paid in full, within ten (10) days after receipt of an invoice therefor.

**14.7 Landlord's Insurance.** Landlord shall take out and maintain in force throughout the term hereof, in a company or companies authorized to do business in the Commonwealth of Massachusetts: (a) property insurance on the Building (exclusive of Tenant's Property, Tenant-Insured Improvements and personal property of, and alterations by, other tenants or occupants) in an amount equal to the full replacement value of the Building (exclusive of foundations and those items set forth in the preceding parenthetical in this sentence), covering fire, vandalism, malicious mischief, extended coverage and so-called "all risk"; (b) rental interruption insurance sufficient to cover at least twelve (12) months of rents from the Property and Landlord's rental losses during such 12-month period, and (c) commercial general liability insurance against claims of bodily injury (including, without limitation, sickness, disease, and death resulting therefrom) and property damage which may be claimed to have occurred from and after the date any of the Tenant Parties shall first enter the Premises arising out of Landlord's operation of the Property of not less than Two Million Dollars (\$2,000,000). Landlord shall also carry umbrella liability coverage in an amount of no less than Ten Million Dollars (\$10,000,000). Such liability policy shall also include contractual liability coverage covering Landlord's liability assumed under this Lease under Section 14.2. The foregoing insurance may be maintained in the form of a blanket policy covering the Property as well as other properties owned by Landlord and Landlord's affiliates. The insurance required pursuant this paragraph shall be effected with insurers with a rating of not less than "A-/VII" in the current *Best's Insurance Reports*, and authorized to do business in the Commonwealth of Massachusetts under valid and enforceable policies. Landlord's insurance policies may include deductibles in an amount no greater than the greater of \$20,000 during the Initial Term of this Lease or commercially reasonable amounts thereafter. Landlord shall deliver to Tenant certificates evidencing the coverage required hereunder upon Tenant's request.

## 15. CASUALTY; TAKING

**15.1 Damage.** If the Premises or any appurtenant areas of the Building or Property necessary to provide access to the Premises or services to, or rights of, Tenant as required hereunder (collectively, the "**Restoration Areas**") are damaged in whole or part because of fire or other insured casualty ("**Casualty**"), or if the Restoration Areas are subject to a taking in connection with the exercise of any power of eminent domain, condemnation, or purchase under threat or in lieu thereof (any of the foregoing, a "**Taking**"), then unless this Lease is terminated in accordance with Section 15.2 below, Landlord shall, subject to the last sentence of this Section 15.1, restore the Restoration Areas to substantially the same condition as existed prior to such Casualty, or in the event of a partial Taking which affects the Restoration Areas, restore the remainder of the Restoration Areas not so Taken to substantially the same condition as is reasonably feasible. If any other portion of the Building is damaged by Casualty or Taking, then Landlord may elect not to restore the same so long as the affected portions of

the Building (other than the Restoration Areas) are otherwise repaired in a manner consistent with first class office and laboratory use. If, in Landlord's reasonable judgment, any element of the Tenant-Insured Improvements can more effectively be restored as an integral part of Landlord's restoration of the Building or the Premises, such restoration shall also be made by Landlord, but at Tenant's sole cost and expense (subject to the amount of insurance proceeds received by Tenant or that would have been

received by Tenant had it been maintaining the coverages required under this Lease). Subject to rights of Mortgagees, Tenant Delays, Legal Requirements then in existence and to delays for adjustment of insurance proceeds or Taking awards, as the case may be, and instances of Force Majeure, Landlord shall use good faith efforts to obtain all required permits therefor and to thereafter promptly commence and diligently prosecute restoration so as to substantially complete restoration of the Premises within twelve (12) months after the date of the Casualty with respect to substantial reconstruction of at least 50% of the Building, or, within one hundred eighty (180) days after the date of the Casualty in the case of restoration of less than 50% of the Building. During such restoration, Landlord shall have the right to relocate Tenant's Parking Spaces in accordance with Section 1.3(b) above. Upon substantial completion of such restoration by Landlord, Tenant shall use diligent efforts to complete restoration of the Premises to substantially the same condition as existed immediately prior to such Casualty or Taking, as the case may be, as soon as reasonably possible. Tenant agrees to cooperate with Landlord in such manner as Landlord may reasonably request to assist Landlord in collecting insurance proceeds due in connection with any Casualty which affects the Restoration Areas. In no event shall Landlord be required to expend more than the Net (hereinafter defined) insurance proceeds Landlord receives for damage to the Premises and/or the Building or the Net Taking award attributable to the Premises and/or the Building. "**Net**" means the insurance proceeds or Taking award actually paid to Landlord (and not paid over to a Mortgagee) less all reasonable third party costs and expenses, including adjusters and attorney's fees, of obtaining the same. In the fiscal year in which a Casualty occurs, there shall be included in Operating Costs Landlord's deductible under its property insurance policy. Except as Landlord may elect pursuant to this Section 15.1, under no circumstances shall Landlord be required to repair any damage to, or make any repairs to or replacements of, any Tenant-Insured Improvements.

## **15.2 Termination Rights.**

(a) **Landlord's Termination Rights.** Landlord may terminate this Lease upon thirty (30) days' prior written notice to Tenant if: (i) any material portion of the Building or all reasonable means of access thereto is taken; (ii) more than thirty-five percent (35%) of the Building is damaged by Casualty; (iii) if the estimated time to complete restoration, as provided to Tenant by Landlord within ninety (90) days following the occurrence of such Casualty (based on a good faith estimate by a qualified general contractor), exceeds twelve (12) months from the date of Casualty; or (iv) if the estimated cost of Landlord's restoration obligations exceeds the amount of the insurance proceeds released by Landlord's Mortgagee for such purpose by more than \$1,000,000. Landlord shall not exercise its termination rights pursuant to this Section 15.2(a) unless Landlord is terminating the leases of all tenants at the Building.

### **(b) Tenant's Termination Rights.**

(i) If (A) this Lease is not terminated and Landlord fails to complete restoration of the Premises, base Building systems serving the same, reasonable means of access to the Premises and the parking areas serving Tenant (collectively, the "**Primary Areas**") within the time frames and subject to the conditions set forth in Section 15.1 above, or (B) if the estimated time to complete restoration of the Primary Areas (as provided pursuant to the immediately preceding paragraph) exceeds the timeframes set forth in Section 15.1 above, then Tenant may terminate this Lease upon thirty (30) days' written notice to Landlord; provided, however, that if Landlord completes such restoration within thirty (30) days after receipt of any such termination notice, such termination notice shall be null and void and this Lease shall continue in full force and effect.

(ii) In addition, if Landlord's Mortgagee does not release sufficient insurance proceeds to cover the cost of Landlord's restoration obligations, then Landlord shall notify Tenant thereof. If such notice by Landlord does not include an agreement by Landlord to pay for the difference between the cost of such restoration and such released insurance proceeds, then Tenant may terminate

this Lease by written notice to Landlord on or before the date that is thirty (30) days after such notice.

(iii) The remedies set forth in this Section 15.2 are Tenant's sole and exclusive rights and remedies based upon Landlord's failure to complete the restoration of the Premises as set forth herein.

(c) **Either Party May Terminate.** In the case of any Casualty or Taking affecting the Premises and occurring during the last twelve (12) months of the Term and the restoration of the Restoration Areas is estimated to take greater than twenty-five percent (25%) of the then-remaining Term, then either Landlord or Tenant shall have the option to terminate this Lease upon thirty (30) days' written notice to the other. If this Lease is terminated as a result of any Casualty or Taking, Landlord shall promptly refund to Tenant any rent and other payments paid prior to such termination but relating to periods from and after such termination. Landlord shall not exercise its termination rights pursuant to this Section 15.2(c) unless Landlord terminates the leases of all tenants at the Building.

(d) **Automatic Termination.** In the case of a Taking of the entire Premises, then this Lease shall automatically terminate as of the date of possession by the Taking authority.

(e) **Rent Abatement.** Following any Casualty or Taking, the Rent under this Lease shall be equitably abated for the period of any restoration of the Premises and a reasonable means of access thereto and a reasonable additional period thereafter for the restoration of any Tenant-Insured Improvements, such abatement to be based on the portion of the Premises rendered untenable on account of the Casualty or Taking.

**15.3 Taking for Temporary Use.** If the Premises are Taken for temporary use, this Lease and Tenant's obligations, including without limitation the payment of Rent, shall continue and Tenant shall be entitled to all of the proceeds of such temporary taking to the extent relating to periods of time within the Term. For purposes hereof, a "**Taking for temporary use**" shall mean a Taking of ninety (90) days or less.

**15.4 Disposition of Awards.** Except for any separate award for Tenant's movable trade fixtures and relocation expenses (provided that the same may not reduce Landlord's award), and unamortized leasehold improvements paid for by Tenant (i.e., in excess of Landlord's Contribution and any other improvements paid for by Landlord), all Taking awards to Landlord or Tenant shall be Landlord's property without Tenant's participation, and Tenant hereby assigns to Landlord Tenant's interest, if any, in such award. Tenant may pursue its own claim against the Taking authority.

**16. ESTOPPEL CERTIFICATE.** Tenant shall at any time and from time to time upon not less than ten (10) business days' prior notice from Landlord, execute, acknowledge and deliver to Landlord a statement in writing certifying that this Lease is unmodified and in full force and effect (or if there have been modifications, that the same is in full force and effect as modified and stating the modifications), and the dates to which Rent has been paid in advance, if any, stating whether or not, to the knowledge of Tenant, Landlord is in default in performance of any covenant, agreement, term, provision or condition contained in this Lease and, if so, specifying each such default, and such other facts as Landlord may reasonably request, it being intended that any such statement delivered pursuant hereto may be relied upon by any prospective purchaser of the Building or of any interest of Landlord therein, any Mortgagee or prospective Mortgagee thereof, any lessor or prospective lessor thereof, any lessee or prospective lessee thereof, or any prospective assignee of any mortgage thereof. Landlord shall at any time and from time to time upon not less than ten (10) business days' prior notice from Tenant execute, acknowledge and deliver to Tenant a statement in writing certifying that this Lease is unmodified and in full force and effect (or if there have been modifications, that the same is in full force and effect as modified and stating the modifications), and the dates to which Rent has been paid in advance, if any, stating whether or not, to

the knowledge of Landlord, Tenant is in default in performance of any covenant, agreement, term, provision or condition contained in this Lease and, if so, specifying each such default, and such other facts as Tenant may reasonably request, it being intended that any such statement delivered pursuant hereto may be relied upon by any prospective Transferee, or any lender or prospective lender of any Tenant. *Time is of the essence with respect to any such requested certificate*, the parties hereby acknowledging the importance of such certificates in mortgage financing arrangements, prospective sales and the like. In the case of any discrepancy between the Lease and the Estoppel Certificate, the Lease shall govern.

## **17. HAZARDOUS MATERIALS**

**17.1 Prohibition.** Except for standard office, cleaning and maintenance supplies used in ordinary amounts and stored in proper containers in compliance with all Environmental Laws, Tenant shall not, without the prior written consent of Landlord, bring or permit to be brought or kept in or on the Premises or elsewhere in the Building or the Property (i) any inflammable, combustible or explosive fluid, material, chemical or substance (except for standard office supplies stored in proper containers and Hazardous Materials in compliance with the following provisions of this Lease, provided that Tenant shall not be entitled to store more than Tenant's Share of inflammable materials allowable pursuant to applicable Legal Requirements at the Building without Landlord being required to hold the license therefor; and (ii) any Hazardous Material (hereinafter defined), other than the types and quantities of Hazardous Materials which are listed on Exhibit 7 attached hereto or otherwise required in the normal operation of Tenant's business ("Tenant's Hazardous Materials"), provided that the same shall at all times be brought upon, kept or used in so-called 'control areas' (the number and size of which shall be reasonably determined by Landlord, but in no event shall the Premises be required to contain less than Tenant's Share of the fire control and chemical storage areas permitted in the Building throughout the Term) and in accordance with all applicable Environmental Laws (hereinafter defined) and prudent environmental practice and (with respect to medical waste and so-called "biohazard" materials) good medical practice. Tenant shall be responsible for assuring that all laboratory uses are adequately and properly vented. On or before each anniversary of the Rent Commencement Date, and additionally at Landlord's request (made no more often than once per Rent Year) in connection with a sale or financing of all or any portion of Landlord's interest in the Property, Tenant shall submit to Landlord an updated list of the types and quantities of Tenant's Hazardous Materials. Landlord shall have the right, from time to time, to inspect the Premises for compliance with the terms of this Section 17.1 subject to the provisions of Section 2.4 of this Lease. Notwithstanding the foregoing, with respect to any of Tenant's Hazardous Materials which Tenant does not properly handle, store or dispose of in compliance with all applicable Environmental Laws (hereinafter defined), prudent environmental practice and (with respect to medical waste and so-called "biohazard materials) good medical practice, Tenant shall, upon written notice from Landlord, no longer have the right to bring such material into the Building or the Property until Tenant has demonstrated, to Landlord's reasonable satisfaction, that Tenant has implemented programs to thereafter properly handle, store or dispose of such material.

**17.2 Environmental Laws.** For purposes hereof, "Environmental Laws" shall mean all laws, statutes, ordinances, rules and regulations of any local, state or federal governmental authority having jurisdiction concerning environmental and human health and safety matters, including but not limited to any discharge by any of the Tenant Parties into the air, surface water, sewers, soil or groundwater of any Hazardous Material (hereinafter defined) whether within or outside the Premises, including, without limitation (a) the Federal Water Pollution Control Act, 33 U.S.C. Section 1251 et seq., (b) the Federal Resource Conservation and Recovery Act, 42 U.S.C. Section 6901 et seq., (c) the Comprehensive Environmental Response, Compensation and Liability Act, 42 U.S.C. Section 9601 et seq., (d) the Toxic Substances Control Act of 1976, 15 U.S.C. Section 2601 et seq., and (e) Chapter 21E of the General Laws of Massachusetts. Tenant, at its sole cost and expense, shall comply with (i) Environmental Laws, and (ii) any rules, requirements and safety procedures of the Massachusetts Department of Environmental Protection, the Town of Lexington and the reasonable requirements of any

insurer of the Building or the Premises (provided that the same are consistent with those applicable to similar uses in similar buildings in the areas) with respect to Tenant's use, storage and disposal of any Hazardous Materials.

**17.3 Hazardous Material Defined.** As used herein, the term "Hazardous Material" means asbestos, oil or any hazardous, radioactive or toxic substance, material or waste or petroleum derivative which is or becomes regulated by any Environmental Law. The term "Hazardous Material" includes, without limitation, oil and/or any material or substance which is (i) designated as a "hazardous substance," "hazardous material," "oil," "hazardous waste" or toxic substance under any Environmental Law.

**17.4 Testing.** If any Mortgagee or governmental authority requires testing to determine whether there has been any release of Hazardous Materials, such testing is required as a result of the acts or omissions of any of the Tenant Parties, and such testing discovers a violation of the provisions of this Section 17 by Tenant, then Tenant shall reimburse Landlord upon demand, as additional rent, for the reasonable third party costs thereof, together with interest at the Default Rate until paid in full. Tenant shall execute affidavits, certifications and the like, as may be reasonably requested by Landlord from time to time concerning Tenant's knowledge and belief concerning the presence of Hazardous Materials in or on the Premises, the Building or the Property.

### **17.5 Indemnity; Remediation.**

(a) Tenant hereby covenants and agrees to indemnify, defend and hold the Landlord Parties harmless from and against any and all Claims against any of the Landlord Parties arising out of contamination of any part of the Property or other adjacent property, which contamination arises as a result of: (i) the presence of Hazardous Material in amounts in excess of Reportable Quantities or Reportable Concentrations (as those terms are defined in Environmental Laws) or in amounts requiring a response action pursuant to any Environmental Law in the Premises, the presence of which is caused by any act or (where Tenant has a duty to act) omission of any of the Tenant Parties, or (ii) from a breach by Tenant of its obligations under this Section 17. This indemnification of the Landlord Parties by Tenant includes, without limitation, reasonable third party costs incurred in connection with any investigation of site conditions or any

cleanup, remedial, removal or restoration work required by any federal, state or local governmental agency or political subdivision because of Hazardous Material present in the soil or ground water on or under the Building based upon the circumstances identified in the first sentence of this Section 17.5. The indemnification and hold harmless obligations of Tenant under this Section 17.5 shall survive the expiration or any earlier termination of this Lease for a period of five years. Without limiting the foregoing, if the presence of any Hazardous Material in the Building or otherwise in the Property in amounts in excess of Reportable Quantities or Reportable Concentrations (as those terms are defined in Environmental Laws) or in amounts requiring a response action pursuant to any Environmental Law is caused by any of the Tenant Parties and results in any contamination of any part of the Property or any adjacent property, Tenant shall promptly take all actions at Tenant's sole cost and expense as are necessary to return the Property and/or the Building or any adjacent property to their condition as of the date of this Lease, provided that Tenant shall first obtain Landlord's approval of such actions, which approval shall not be unreasonably withheld, conditioned or delayed so long as such actions, in Landlord's reasonable discretion, would not potentially have any adverse effect on the Property, and, in any event, Landlord shall not withhold its approval of any proposed actions which are required by applicable Environmental Laws. For the avoidance of doubt, the parties acknowledge that Tenant's Remediation with respect to the Property shall not contain any requirement that Tenant remediate any contamination to levels or standards more stringent than those associated with the Permitted Uses. The provisions of this Section 17.5 shall survive the expiration or earlier termination of the Lease for a period of five years.

(b) Without limiting the obligations set forth in Section 17.5(a) above, if any Hazardous Material is in, on, under, at or about the Building or the Property as a result of the acts or omissions of any of the Tenant Parties and results in any contamination of any part of the Property or any adjacent property that is in violation of any applicable Environmental Law or that requires the performance of any response action pursuant to any Environmental Law, Tenant shall promptly take all actions at Tenant's sole cost and expense as are necessary to reduce such Hazardous Material to amounts below any applicable Reportable Quantity, any applicable Reportable Concentration and any other applicable standard set forth in Environmental Laws; provided that Tenant shall first obtain Landlord's written approval of such actions, which approval shall not be unreasonably withheld, conditioned or delayed so long as such actions would not be reasonably expected to have an adverse effect on the market value or utility of the Property for the Permitted Uses, and in any event, Landlord shall not withhold its approval of any proposed actions which are required by applicable Environmental Laws (such approved actions, "**Tenant's Remediation**").

(c) In the event that Tenant fails to complete Tenant's Remediation prior to the end of the Term, then:

(i) provided that Landlord does not permit occupancy of the Premises prior to the completion of Tenant's Remediation, until the completion of Tenant's Remediation (as evidenced by the certification of Tenant's Licensed Site Professional (as such term is defined by applicable Environmental Laws), who shall be reasonably acceptable to Landlord) (the "**Remediation Completion Date**"), Tenant shall pay to Landlord, with respect to the portion of the Premises which reasonably cannot be occupied by a new tenant until completion of Tenant's Remediation, (A) Additional Rent on account of Operating Costs and Taxes and (B) Base Rent in an amount equal to the greater of (1) the fair market rental value of such portion of the Premises (determined in substantial accordance with the process described in Section 1.2 above), and (2) Base Rent attributable to such portion of the Premises in effect immediately prior to the end of the Term; and

(ii) Tenant shall maintain responsibility for Tenant's Remediation and Tenant shall complete Tenant's Remediation as soon as reasonably practicable in accordance with Environmental Laws. If Tenant does not diligently pursue completion of Tenant's Remediation, Landlord shall have the right to either (A) assume control for overseeing Tenant's Remediation, in which event Tenant shall pay all reasonable costs and expenses of Tenant's Remediation (it being understood and agreed that all costs and expenses of Tenant's Remediation incurred pursuant to contracts entered into by Tenant shall be deemed reasonable) within thirty (30) days of demand therefor (which demand shall be made no more often than monthly), and Landlord shall be substituted as the party identified on any governmental filings as the party responsible for the performance of such Tenant's Remediation or (B) require Tenant to maintain responsibility for Tenant's Remediation, in which event Tenant shall complete Tenant's Remediation as soon as reasonably practicable in accordance with Environmental Laws, it being understood that Tenant's Remediation shall not contain any requirement that Tenant remediate any contamination to levels or standards more stringent than those associated with the Property's current office, research and development, laboratory, and vivarium uses.

(d) The provisions of this Section 17.5 shall survive the expiration or earlier termination of this Lease for a period of five years.

**17.6 Disclosures.** In connection with bringing any Hazardous Material into any part of the Property, Tenant shall maintain and, upon Landlord's request, deliver to Landlord (subject to the provisions of Section 26.16) the following information with respect thereto: (a) a description of handling, storage, use and disposal procedures; (b) all non-proprietary plans or disclosures and/or emergency response plans which Tenant has prepared, including without limitation Tenant's Spill Response Plan,

and all plans which Tenant is required to supply to any governmental agency or authority pursuant to any Environmental Laws; (c) copies of all Required Permits relating thereto; and (d) other non-proprietary information reasonably requested by Landlord.

**17.7 Removal.** Tenant shall be responsible, at its sole cost and expense, for Hazardous Material and other biohazard disposal services for the Premises. Such services shall be performed by contractors reasonably acceptable to Landlord and on a sufficient basis to ensure that the Premises are at all times kept neat, clean and free of waste, Hazardous Materials and biohazards except in appropriate, specially marked containers reasonably approved by Landlord.

**17.8 Landlord's Obligations.** Landlord shall, at its sole cost and expense, comply with all Environmental Laws with respect to the existence of Hazardous Materials in, on or at the Property as of the Execution Date. Landlord agrees to indemnify, defend and hold the Tenant Parties harmless from and against any and all Claims against any of the Tenant Parties arising out of (a) the existence of Hazardous Materials in, on, under or at the Property as of the Execution Date except to the extent that any of the Tenant Parties exacerbates a release of the same that occurred prior to the Execution Date, and (b) the release by any of the Landlord Parties of any Hazardous Materials in, on, under or at the Property in excess of Reportable Quantities or Reportable Concentrations. This indemnification of the Tenant Parties by Landlord includes, without limitation, reasonable third party costs incurred in connection with any investigation of site conditions or any cleanup, remedial, removal or restoration work required by any federal, state or local governmental agency or political subdivision because of Hazardous Material present in the soil or ground water on or under the Building based upon the circumstances identified in the first sentence of this Section 17.8. The indemnification and hold harmless obligations of Landlord under this Section 17.8 shall survive the expiration or any earlier termination of this Lease for a period of five years. Without limiting the foregoing, if the presence of any Hazardous Material in the Building or otherwise in the Property in amounts in excess of Reportable Quantities or Reportable Concentrations (as those terms are defined in Environmental Laws) or in amounts requiring a response action pursuant to any Environmental Law is caused by any of the Landlord Parties and results in any contamination of any part of the Property or any adjacent property, Landlord shall promptly take all actions at Landlord's sole cost and expense as are necessary to remediate the same to levels below Reportable Quantities or Reportable

Concentrations. In addition, if the presence of any Hazardous Material in the Building or otherwise in the Property in amounts in excess of Reportable Quantities or Reportable Concentrations (as those terms are defined in Environmental Laws) or in amounts requiring a response action pursuant to any Environmental Law is caused by any of the other tenants of the Building and results in any contamination of any part of the Building, Landlord shall promptly take, or cause to be taken, all actions at Landlord's sole cost and expense as are necessary to remediate the same to levels below Reportable Quantities or Reportable Concentrations. For the avoidance of doubt, the parties acknowledge that Landlord shall not be required to remediate any contamination to levels or standards more stringent than those associated with the Permitted Uses.

## 18. RULES AND REGULATIONS.

**18.1 Rules and Regulations.** Tenant will faithfully observe and comply with all reasonable rules and regulations promulgated from time to time with respect to the Building, the Property and construction within the Property of which Tenant has prior written notice (collectively, the "**Rules and Regulations**"). The current version of the Rules and Regulations is attached hereto as Exhibit 8. In the case of any conflict between the provisions of this Lease and any future rules and regulations, the provisions of this Lease shall control. Landlord agrees to enforce the Rules and Regulations against all tenants of the Building in a uniform and non-discriminatory manner, but Landlord shall not be liable to Tenant for violation of the same by any other tenant, its servants, employees, agents, contractors, visitors, invitees or licensees.

**18.2 Energy Conservation.** Landlord may institute upon written notice to Tenant such

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reasonable policies, programs and measures as may be necessary, required, or expedient for the conservation and/or preservation of energy or energy services (collectively, the "**Conservation Program**"), provided however, that the Conservation Program does not, by reason of such policies, programs and measures, reduce the level of energy or energy services being provided to the Premises below the level of energy or energy services then being provided in comparable combination laboratory, research and development and office buildings in the vicinity of the Premises, or as may be necessary or required to comply with Legal Requirements or standards or the other provisions of this Lease. Upon receipt of such notice, Tenant shall comply with the Conservation Program provided that Tenant shall have no obligation to incur any additional costs (other than de minimis costs) or alter any of its operations in the Premises in a manner that could adversely affect Tenant's business to do so. Landlord shall consult with Tenant prior to implementing any Conservation Program.

**18.3 Recycling.** Upon written notice, Landlord may establish policies, programs and measures for the recycling of paper, products, plastic, tin and other materials (a "**Recycling Program**"). Upon receipt of such notice, Tenant will comply with the Recycling Program at Tenant's sole cost and expense.

## 19. LAWS AND PERMITS.

**19.1 Legal Requirements.** Tenant shall be responsible at its sole cost and expense for complying with (and keeping the Premises in compliance with) all Legal Requirements which are applicable to Tenant's particular use or occupancy of, or Alterations made by or on behalf of Tenant to, the Premises, as opposed to office and laboratory use, generally. Tenant shall furnish all data and information to governmental authorities, with a copy of any non-proprietary information to Landlord, as required in accordance with Legal Requirements as they relate to Tenant's use or occupancy of the Premises or the Building. If Tenant receives notice of any violation of Legal Requirements applicable to the Premises or the Building, it shall give prompt notice thereof to Landlord (provide that failure to do so shall not be deemed to be a default hereunder). Nothing contained in this Section 19.1 shall be construed to expand the uses permitted hereunder beyond the Permitted Uses. Landlord shall comply with any Legal Requirements and with any direction of any public office or officer relating to the maintenance or operation of the Building as a combination laboratory, research and development and office building, and the costs so incurred by Landlord shall be included in Operating Costs in accordance with, and subject to the limitations set forth in, the provisions of Section 5.2.

**19.2 Required Permits.** Tenant shall, at Tenant's sole cost and expense, use diligent good faith efforts to apply for, seek and obtain all necessary state and local licenses, permits and approvals needed for the operation of Tenant's business and/or Tenant's Rooftop Equipment (collectively, the "**Required Permits**"), as soon as reasonably possible, and in any event shall not undertake any operations or use of Tenant's Rooftop Equipment unless all applicable Required Permits are in place. Tenant shall thereafter maintain all Required Permits. Tenant, at Tenant's expense, shall at all times comply with the terms and conditions of each such Required Permit. Landlord shall cooperate with Tenant, at Tenant's sole cost and expense, in connection with its application for Required Permits.

**19.3 Traffic Management.** Tenant acknowledges that the Property is subject to a traffic mitigation and/or management plan, a copy of which is attached hereto as Exhibit 10 (the "**PTDM**"). Tenant agrees not to violate the terms of the PTDM applicable to tenants of the Building. Tenant shall, at Tenant's sole expense, for so long as the PTDM remains applicable to the Property, (a) participate in the Hartwell Avenue Transportation Management Association, (b) to the extent required by the PTDM, allow employees at the Premises to set-aside pre-tax funds as allowable under the Commuter Choice provision of the Federal tax code, and (c) reasonably cooperate with Landlord in (i) connection with Landlord's reporting obligations under the PTDM and any amendments thereto, and (ii) encouraging employees to avoid vehicle trips at peak commuting hours and to seek alternate modes of transportation. The costs incurred by Landlord in connection with compliance with the PTDM shall be included in Operating

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Costs.

## 20. DEFAULT

**20.1 Events of Default.** The occurrence of any one or more of the following events shall constitute an "**Event of Default**" hereunder by Tenant:

(a) If Tenant fails to make any payment of Rent or any other payment required hereunder, as and when due, and such failure shall continue for a period of five (5) days after notice thereof from Landlord to Tenant; provided, however, an Event of Default shall occur hereunder without any obligation of Landlord to give any notice if (i) Tenant fails to make any payment within five (5) days after the due date therefor, and (ii) Landlord has given Tenant written notice under this Section 20.1(a) on more than one (1) occasion during the twelve (12) month interval preceding such failure by Tenant;

(b) If Tenant shall fail to execute and deliver to Landlord an estoppel certificate pursuant to Section 16 above or a subordination and attornment agreement pursuant to Section 22 below, within the timeframes set forth therein, following notice and an additional five (5) day period in which to cure such failure;

(c) If Tenant shall fail to maintain any insurance required hereunder;

(e) If Tenant shall fail to restore the Security Deposit to its original amount or deliver a replacement Letter of Credit as required under Section 7 above;

(f) If Tenant shall make a Transfer in violation of the provisions of Section 13 above, or if any event shall occur or any contingency shall arise whereby this Lease, or the term and estate thereby created, would (by operation of law or otherwise) devolve upon or pass to any person, firm or corporation other than Tenant, except as expressly permitted under Section 13 hereof, and Tenant does not cure such default with ten (10) days following notice from Landlord;

(g) The failure by Tenant to observe or perform any of the covenants or provisions of this Lease to be observed or performed by Tenant, other than as specified above, and such failure continues for more than thirty (30) days after notice thereof from Landlord; provided, further, that if the nature of Tenant's default is such that more than thirty (30) days are reasonably required for its cure, then Tenant shall not be deemed to be in default if Tenant shall commence such cure within said thirty (30) day period and thereafter diligently prosecute such cure to completion;

(h) If Tenant shall make an admission in writing of Tenant's inability to pay its debts generally as they become due;

(i) If Tenant shall make an assignment or trust mortgage, or other conveyance or transfer of like nature, of all or a substantial part of its property for the benefit of its creditors,

(j) If an attachment on mesne process, on execution or otherwise, or other legal process shall issue against Tenant or a material property of Tenant and a sale of any of its assets shall be held thereunder;

(k) If the leasehold hereby created shall be taken on execution or by other process of law and shall not be revested in Tenant within sixty (60) days thereafter;

(l) If a receiver, sequesterer, trustee or similar officer shall be appointed by a court of competent jurisdiction to take charge of all or any part of Tenant's Property and such appointment shall not be vacated within sixty (60) days;

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(m) If any proceeding shall be instituted by or against Tenant pursuant to any of the provisions of any Act of Congress or State law relating to bankruptcy, reorganizations, arrangements, compositions or other relief from creditors, and, in the case of any proceeding instituted against it, if Tenant shall fail to have such proceedings dismissed within sixty (60) days or if Tenant is adjudged bankrupt or insolvent as a result of any such proceeding; or

(n) If there occurs an event expressly referred to as a material default by Guarantor under the Guaranty.

Wherever "Tenant" is used in subsections (h), (i), (j), (l) or (m) of this Section 20.1, it shall be deemed to include any guarantor of any of Tenant's obligations under this Lease, including Guarantor.

**20.2 Remedies.** Upon an Event of Default, Landlord may, by notice to Tenant, elect to terminate this Lease; and thereupon (and without prejudice to any remedies which might otherwise be available for arrears of Rent or preceding breach of covenant or agreement and without prejudice to Tenant's liability for damages as hereinafter stated), upon the giving of such notice, this Lease shall terminate as of the date specified therein as though that were the Expiration Date. Upon such termination, Landlord shall have the right to utilize the Security Deposit or draw down the entire Letter of Credit, as applicable, and apply the proceeds thereof to its damages hereunder. Without being taken or deemed to be guilty of any manner of trespass or conversion, and without being liable to indictment, prosecution or damages therefor, Landlord may, by lawful process, enter into and upon the Premises (or any part thereof in the name of the whole); repossess the same, as of its former estate; and expel Tenant and those claiming under Tenant. The words "re-entry" and "re-enter" as used in this Lease are not restricted to their technical legal meanings.

### **20.3 Damages - Termination.**

(a) Upon the termination of this Lease under the provisions of this Section 20, Tenant shall pay to Landlord Rent up to the time of such termination, shall continue to be liable for any preceding breach of covenant, and in addition, shall pay to Landlord as damages, at the election of Landlord, either:

(i) the amount (discounted to present value at the rate of eight percent (8%) per annum) by which, at the time of the termination of this Lease (or at any time thereafter if Landlord shall have initially elected damages under Section 20.3(a)(ii) below), (x) the aggregate of Rent projected over the period commencing with such termination and ending on the Expiration Date, exceeds (y) the aggregate projected rental value of the Premises for such period, taking into account a reasonable time period during which the Premises shall be unoccupied, plus all Reletting Costs (hereinafter defined); or

(ii) amounts equal to Rent which would have been payable by Tenant had this Lease not been so terminated, payable upon the due dates therefor specified herein following such termination and until the Expiration Date, *provided, however*, if Landlord shall re-let the Premises during such period, that Landlord shall credit Tenant with the net rents received by Landlord from such re-letting, such net rents to be determined by first deducting from the gross rents as and when received by Landlord from such re-letting the expenses incurred or paid by Landlord in terminating this Lease, as well as the expenses of re-letting, including altering and preparing the Premises for new tenants, brokers' commissions, and all other similar and dissimilar expenses properly chargeable against the Premises and the rental therefrom (collectively, "**Reletting Costs**"), it being understood that any such re-letting may be for a period equal to or shorter or longer than the remaining Term; and *provided, further*, that (x) in no event shall Tenant be entitled to receive any excess of such net rents over the sums payable by Tenant to Landlord hereunder and (y) in no event shall Tenant be entitled in any suit for the collection of damages pursuant to this Section 20.3(a)(ii) to a credit in respect of any net rents from a re-letting except to the extent that such net rents are actually received by Landlord prior to the commencement of such suit. If

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the Premises or any part thereof should be re-let in combination with other space, then proper apportionment on a square foot area basis shall be made of the rent received from such re-letting and of the expenses of re-letting.

(b) In calculating the amount due under Section 20.3(a)(i), above, there shall be included, in addition to the Base Rent, all other considerations agreed to be paid or performed by Tenant, including without limitation Tenant's Share of Operating Costs and Taxes, on the assumption that all such amounts and considerations would have increased at the rate of five percent (5%) per annum for the balance of the full term hereby granted.

(c) Suit or suits for the recovery of such damages, or any installments thereof, may be brought by Landlord from time to time at its election, and nothing contained herein shall be deemed to require Landlord to postpone suit until the date when the Term would have expired if it had not been terminated hereunder.

(d) Nothing herein contained shall be construed as limiting or precluding the recovery by Landlord against Tenant of any sums or damages to which, in addition to the damages particularly provided above, Landlord may lawfully be entitled by reason of any Event of Default hereunder. After a termination of this Lease pursuant to this Section 20, Landlord will use reasonable efforts to relet the Premises after Tenant vacates the Premises; however, the marketing of the Premises in a manner similar to the manner in which Landlord markets other premises in the Building shall be deemed to have satisfied Landlord's obligation to use "reasonable efforts." In no event shall Landlord be required to (i) solicit or entertain negotiations with any other prospective tenants for the Premises unless and until Landlord obtains full and complete possession of the Premises, including the final and unappealable legal right to relet the Premises free of any claim of Tenant, (ii) lease the Premises to a tenant whose proposed use, in Landlord's reasonable judgment, will be unacceptable, (iii) relet the Premises prior to leasing any other vacant space in the Building, suitable for the use of the prospective tenant, (iv) lease the Premises for a rental rate less than the current fair market rent then prevailing for similar space in the Building, or (v) enter into a lease with any proposed tenant that does not have, in Landlord's reasonable opinion, sufficient financial wherewithal and resources to satisfy its financial obligations under the prospective lease. Landlord shall be entitled to take into account in connection with any such reletting of the Premises all relevant factors which would be taken into account by a sophisticated landlord in securing a replacement tenant for the Premises including the first class quality of the Property, and the financial responsibility of any such replacement tenant.

(e) In lieu of any other damages or indemnity and in lieu of full recovery by Landlord of all sums payable under all the foregoing provisions of this Section 20.3, Landlord may, by written notice to Tenant, at any time after this Lease is terminated under any of the provisions herein contained or is otherwise terminated for breach of any obligation of Tenant and before such full recovery, elect to recover, and Tenant shall thereupon pay, as liquidated damages, an amount equal to the aggregate of (x) an amount equal to the lesser of (1) Rent accrued under this Lease in the twelve (12) months immediately prior to such termination, or (2) Rent payable during the remaining months of the Term if this Lease had not been terminated, plus (y) the amount of Rent accrued and unpaid at the time of termination, less (z) the amount of any recovery by Landlord under the foregoing provisions of this Section 20.3 up to the time of payment of such liquidated damages.

**20.4 Landlord's Self-Help.** If Tenant shall default in the performance of any covenant on Tenant's part to be performed in this Lease contained, including without limitation the obligation to maintain the Premises in the required condition pursuant to Section 10.1 above, Landlord may, after the expiration of the cure periods set forth in Section 20.1 above and upon reasonable advance notice (except that in an emergency or if any such default constitutes a violation of Legal Requirements no notice shall be required and Landlord shall not be required to wait for the expiration of such cure periods), immediately, or at any time thereafter, perform the same for the account of Tenant. Tenant shall pay to

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Landlord upon demand therefor any reasonable costs incurred by Landlord in connection therewith, together with interest at the Default Rate until paid in full.

**20.5 Waiver of Redemption, Statutory Notice and Grace Periods.** Tenant does hereby waive and surrender all rights and privileges which it might have under or by reason of any present or future Legal Requirements to redeem the Premises or to have a continuance of this Lease for the Term hereby demised after being dispossessed or ejected therefrom by process of law or under the terms of this Lease or after the termination of this Lease as herein provided. Except to the extent prohibited by Legal Requirements, any statutory notice and grace periods provided to Tenant by law are hereby expressly waived by Tenant.

**20.6 Landlord's Remedies Not Exclusive.** The specified remedies to which Landlord may resort hereunder are cumulative and are not intended to be exclusive of any remedies or means of redress to which Landlord may at any time be lawfully entitled, and Landlord may invoke any remedy (including the remedy of specific performance) allowed at law or in equity as if specific remedies were not herein provided for.

**20.7 No Waiver.** Either party's failure to seek redress for violation, or to insist upon the strict performance, of any covenant or condition of this Lease, or any of the Rules and Regulations promulgated hereunder, shall not prevent a subsequent act, which would have originally constituted a violation, from having all the force and effect of an original violation. The receipt by Landlord of Rent with knowledge of the breach of any covenant of this Lease shall not be deemed a waiver of such breach. The failure of Landlord to enforce any of such Rules and Regulations against Tenant and/or any other tenant in the Building shall not be deemed a waiver of any such Rules and Regulations. No provisions of this Lease shall be deemed to have been waived by either party unless such waiver be in writing signed by such party. No payment by Tenant or receipt by Landlord of a lesser amount than the Rent herein stipulated shall be deemed to be other than on account of the stipulated Rent, nor shall any endorsement or statement on any check or any letter accompanying any check or payment as Rent be deemed an accord and satisfaction, and Landlord may accept such check or payment without prejudice to Landlord's right to recover the balance of such Rent or pursue any other remedy in this Lease provided.

**20.8 Restrictions on Tenant's Rights.** During the continuation of any monetary or material non-monetary Event of Default, (a) Landlord shall not be obligated to provide Tenant with any notice pursuant to Sections 2.3 and 2.4 above; and (b) Tenant shall not have the right to make, nor to request Landlord's consent or approval with respect to, any Alterations or Transfers.

## **20.9 Landlord Default.**

(a) Notwithstanding anything to the contrary contained in the Lease, Landlord shall in no event be in default in the performance of any of Landlord's obligations under this Lease unless Landlord shall have failed to perform such obligations within thirty (30) days (or such additional time as is reasonably required to correct any such default, provided Landlord commences cure within 30 and thereafter diligently prosecute such cure to completion) after notice by Tenant to Landlord properly specifying wherein Landlord has failed to perform any such obligation. Except as expressly set forth in this Lease, Tenant shall not have the right to terminate or cancel this Lease or to withhold rent or to set-off or deduct any claim for damages against rent as a result of any default by Landlord or breach by Landlord of its covenants or any warranties or promises hereunder, except in the case of a wrongful eviction of Tenant from the Premises (constructive or actual) by Landlord, unless same continues after notice to Landlord thereof and an opportunity for Landlord to cure the same as set forth above. In addition, other than as set forth in Section 9.6(b), Tenant shall not assert any right to deduct the cost of repairs or any monetary claim against Landlord from rent thereafter due and payable under this Lease except as expressly set forth in Section 20.9(b) below.

(b) If Landlord is in default (determined in accordance with Section 20.9(a) above) under any provision of this Lease other than Section 3 hereof (it being understood and agreed that Tenant's remedies for Landlord's default of Landlord's Section 3 obligations are set forth in said Section 3), or if Landlord's failure to perform any of its obligations hereunder poses an imminent risk of damage or injury to persons or property or constitutes a violation of Legal Requirements and if such default or failure, as the case may be, materially adversely affects Tenant's ability to operate its business in the ordinary course in accordance with the terms of this Lease, then Tenant shall have the right to cure such default or perform such obligation which Landlord failed to perform, as the case may be, on Landlord's behalf (provided that Tenant shall not violate or render void any warranties maintained by Landlord of which Tenant has prior written notice, and in no event shall any such cure affect any other tenant in the Building), in which event Landlord shall reimburse Tenant within thirty (30) days after receipt of a reasonably detailed invoice for all reasonable costs and expenses incurred by Tenant in connection therewith, together with interest at the Default Rate. Tenant's self-help rights under this Section 20.9(b) shall be exercised by Tenant only (i) with respect to conditions that materially adversely affect Tenant's ability to operate its business in the ordinary course in accordance with the terms of this Lease, and (ii) except in the event of an emergency or a violation of Legal Requirements (in either of which events Tenant shall provide notice to Landlord's designated emergency contact, which notice may be by e-mail or oral, which contact information shall be provided in writing to Tenant and may be changed by Landlord by written notice from time to time), after Tenant has provided Landlord with notice of Tenant's intention to exercise such right (which notice shall be delivered in an envelope that conspicuously states the following in bold caps: "**TENANT NOTICE OF INTENTION TO EXERCISE SELF-HELP**") and which notice shall include an explicit statement that such notice is a notice delivered pursuant to this Section 20.9 and Landlord's failure to perform the specified obligation will trigger the provisions of this Section 20.9, and which notice shall include a copy of the default notice delivered pursuant to Section 20.9(a) above), and Landlord has failed to commence action to remedy the condition complained of within ten (10) days after its receipt of such notice (or if Landlord commences to do the act required within such ten (10) day period but fails to proceed diligently thereafter). The provisions of this Section 20.9(b) are personal to uniQure, Inc. and its Successor(s). If Landlord fails to reimburse Tenant for Tenant's costs incurred pursuant to this Section 20.9(b) within the aforementioned 30 day period, then Tenant may send Landlord a notice in an envelope that conspicuously states the following in bold caps: "**TENANT NOTICE OF INTENTION TO EXERCISE OFF-SET**" and which notice shall include an explicit statement that such notice is a notice delivered pursuant to this Section 20.9(b) and describing Landlord's failure to make such reimbursement and, if Landlord fails to reimburse Tenant within ten (10) days following delivery of such notice, then Tenant may off-set such amounts, together with interest at the Default Rate from the date incurred by Tenant, against the Rent due hereunder until Tenant is paid in full (provided that such off-set shall not exceed 15% of the Base Rent due from Tenant in any one month).

## 21. SURRENDER; ABANDONED PROPERTY; HOLD-OVER

### 21.1 Surrender

(a) Upon the expiration or earlier termination of the Term, Tenant shall (i) peaceably quit and surrender to Landlord the Premises (including without limitation all fixed lab benches, fume hoods, electric, plumbing, heating and sprinkling systems, fixtures and outlets, vaults, paneling, molding, shelving, radiator enclosures, cork, rubber, linoleum and composition floors, ventilating, silencing, air conditioning and cooling equipment therein) broom clean, in the condition in which Tenant was obligated to maintain the same excepting only ordinary wear and tear and damage by fire or other Casualty; (ii) remove all of Tenant's Property, all autoclaves and cage washers and, to the extent specified by Landlord in accordance with Section 11.1 above, Alterations made by Tenant; and (iii) repair any damages to the Premises or the Building caused by the installation or removal of Tenant's Property and/or such Alterations. Tenant's obligations under this Section 21.1(a) shall survive the expiration or earlier termination of this Lease.

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(b) At least thirty (30) days prior to the expiration of the Term (or, if applicable, within five (5) business days after any earlier termination of this Lease), Tenant shall deliver to Landlord a narrative description of the actions proposed (or required by any Legal Requirements) to be taken by Tenant in order to render the Premises (including any Alterations permitted or required by Landlord to remain therein) free of Hazardous Materials in excess of Reportable Quantities or Reportable Concentrations in compliance with Legal Requirements and to the extent subject to regulation under 105 CMR 120.000 et seq., released for unrestricted use in accordance with 105 CMR 120.243 and 105 CMR 120.245 (the "**Surrender Plan**"). The Surrender Plan (i) shall be accompanied by a current list of (A) all Required Permits held by or on behalf of any Tenant Party with respect to Hazardous Materials in, on, under, at or about the Premises, and (B) Tenant's Hazardous Materials used by or on behalf of any Tenant Party in, on, under, at or about the Premises, and (ii) shall be subject to the reasonable review and approval of Landlord's environmental consultant. In connection with review and approval of the Surrender Plan, upon request of Landlord, Tenant shall deliver to Landlord or its consultant such additional non-proprietary information concerning the use of and operations within the Premises as Landlord shall reasonably request. On or before the expiration of the Term (or within thirty (30) days after any earlier termination of this Lease, during which period Tenant's use and occupancy of the Premises shall be governed by Section 21.3 below), Tenant shall deliver to Landlord a certification from a qualified third-party environmental professional reasonably approved by Landlord or an industrial hygienist reasonably acceptable to Landlord certifying that the Premises do not contain any Hazardous Materials in excess of Reportable Quantities or Reportable Concentrations on account of the use of Hazardous Materials by any Tenant Party and evidence that the approved Surrender Plan shall have been satisfactorily completed by a contractor reasonably acceptable to Landlord, and Landlord shall have the right, subject to reimbursement at Tenant's expense as set forth below if Tenant is not in compliance with the terms of this subparagraph, to cause Landlord's environmental consultant to inspect the Premises and perform such additional procedures as may be deemed reasonably necessary to confirm that the Premises are, as of the expiration of the Term (or, if applicable, the date which is thirty (30) days after any earlier termination of this Lease), free of Hazardous Materials to the extent set forth above and otherwise available for unrestricted use and occupancy for the Permitted Use. Landlord shall have the unrestricted right to deliver the Surrender Plan and any report by Landlord's environmental consultant with respect to the surrender of the Premises to third parties; however, only the Landlord Parties and Landlord's lender(s) shall be entitled to rely on the Surrender Report. If Tenant shall fail to prepare or submit a Surrender Plan approved by Landlord, or if Tenant shall fail to complete the approved Surrender Plan, or if such Surrender Plan, whether or not approved by Landlord, shall fail to adequately address the use of Hazardous Materials by any of the Tenant Parties in, on, at, under or about the Premises, Landlord shall have the right to take any such actions as Landlord may deem reasonable or appropriate to assure that the Premises and the Property are surrendered in the condition required hereunder, the reasonable third party cost of which actions shall be reimbursed by Tenant as Additional Rent upon demand. Tenant's obligations under this Section 21.1(b) shall survive the expiration or earlier termination of the Term for a period of five (5) years.

(c) No act or thing done by Landlord during the Term shall be deemed an acceptance of a surrender of the Premises, and no agreement to accept such surrender shall be valid, unless in writing signed by Landlord. Unless otherwise agreed by the parties in writing, no employee of Landlord or of Landlord's agents shall have any power to accept the keys of the Premises prior to the expiration or earlier termination of this Lease. The delivery of keys to any employee of Landlord or of Landlord's agents shall not operate as a termination of this Lease or a surrender of the Premises.

**21.2 Abandoned Property.** After the expiration or earlier termination hereof, if Tenant fails to remove any property from the Building or the Premises which Tenant is obligated by the terms of this Lease to remove within five (5) business days after written notice from Landlord, such property (the "**Abandoned Property**") shall be conclusively deemed to have been abandoned, and may either be retained by Landlord as its property or sold or otherwise disposed of in such manner as Landlord may see fit. If any item of Abandoned Property shall be sold, Tenant hereby agrees that Landlord may receive and



retain the proceeds of such sale and apply the same, at its option, to the expenses of the sale, the cost of moving and storage, any damages to which Landlord may be entitled under Section 20 hereof or pursuant to law, and to any arrears of Rent.

**21.3 Holdover.** If any of the Tenant Parties holds over after the end of the Term, Tenant shall be deemed a tenant-at-sufferance subject to the provisions of this Lease; provided that whether or not Landlord has previously accepted payments of Rent from Tenant, (i) Tenant shall pay Base Rent at 150% of the last applicable rate of Base Rent for the first 30 days of such holdover and thereafter 200% of the last applicable rate of Base Rent payable during the Term, which such Base Rent shall be prorated on a per diem basis for partial months, (ii) Tenant shall continue to pay to Landlord all additional rent, and (iii) if such holdover continues for more than 30 days, Tenant shall be liable for all actual damages, including without limitation lost business and consequential damages, incurred by Landlord as a result of such holding over, Tenant hereby acknowledging that Landlord may need the Premises after the end of the Term for other tenants and that the damages which Landlord may suffer as the result of Tenant's holding over cannot be determined as of the Execution Date. Nothing contained herein shall grant Tenant the right to holdover after the expiration or earlier termination of the Term.

## 22. MORTGAGEE RIGHTS

**22.1 Subordination.** Tenant's rights and interests under this Lease shall be (i) subject and subordinate to any ground lease, overleases, mortgage, deed of trust, or similar instrument covering the Premises, the Building and/or the Land and to all advances, modifications, renewals, replacements, and extensions thereof (each of the foregoing, a "**Mortgage**") so long as the applicable Mortgagee and Tenant execute a commercially reasonable subordination, non-disturbance and attornment agreement ("**SNDA**"), (the form of SNDA attached hereto as Exhibit 13 being deemed commercially reasonable) or (ii) if any Mortgagee elects, prior to the lien of any present or future Mortgage. Tenant further shall attorn to and recognize any successor landlord, whether through foreclosure or otherwise, as if the successor landlord were the originally named landlord. Tenant agrees to execute, acknowledge and deliver (and to cause Guarantor to execute, acknowledge and deliver) such instruments, confirming such subordination and attornment, within fifteen (15) days of request therefor. Simultaneously with the execution and delivery of this Lease, Landlord, Tenant and Landlord's current Mortgagee shall enter into an SNDA in form reasonably acceptable to such parties.

**22.2 Notices.** Subject to the terms of any SNDA, Tenant shall give each Mortgagee of which Tenant has been provided prior written notice the same notices given to Landlord concurrently with the notice to Landlord, and each Mortgagee shall have a reasonable opportunity thereafter concurrent with Landlord's cure period but including such additional time required to take possession of the Premises if such possession is reasonably necessary to cure such default, but not to exceed 180 days in the aggregate) to cure a Landlord default, and Mortgagee's curing of any of Landlord's default shall be treated as performance by Landlord.

### 22.3 Intentionally Omitted.

**22.4 Mortgage Liability.** Subject to the terms of any SNDA, Tenant acknowledges and agrees that if any Mortgage shall be foreclosed, (a) the liability of the Mortgagee and its successors and assigns shall exist only so long as such Mortgagee or purchaser is the owner of the Premises, and such liability shall not continue or survive after further transfer of ownership; and (b) such Mortgagee and its successors or assigns shall not be (i) liable for any act or omission of any prior lessor under this Lease; (ii) liable for the performance of Landlord's covenants pursuant to the provisions of this Lease which arise and accrue prior to such entity succeeding to the interest of Landlord under this Lease or acquiring such right to possession; (iii) subject to any offsets or defense which Tenant may have at any time against Landlord (other than Tenant's express offset rights under Sections 3.2, 3.4(d), 9.6(b) and 20.9(b) of this

Lease); (iv) bound by any base rent or other sum which Tenant may have paid previously for more than one (1) month; or (v) liable for the performance of any covenant of Landlord under this Lease which is capable of performance only by the original Landlord, provided that in no event shall the foregoing clauses (i)-(iii) or (v) relieve any such Mortgagee and its successors and assigns from ongoing obligations under this Lease following the date of such succession.

**23. QUIET ENJOYMENT.** Landlord covenants that so long as Tenant keeps and performs each and every covenant, agreement, term, provision and condition herein contained on the part and on behalf of Tenant to be kept and performed, subject to applicable notice and cure periods, Tenant shall peaceably and quietly hold, occupy and enjoy the Premises during the Term from and against the claims of all persons lawfully claiming by, through or under Landlord subject, nevertheless, to the covenants, agreements, terms, provisions and conditions of this Lease, and any matters of record set forth on Exhibit 12.

**24. NOTICES.** Any notice, consent, request, bill, demand or statement hereunder (each, a "**Notice**") by either party to the other party shall be in writing and shall be deemed to have been duly given when either delivered by hand or by nationally recognized overnight courier (in either case with evidence of delivery or refusal thereof) addressed as follows:

If to Landlord: King 113 Hartwell LLC  
c/o King Street Properties  
255 Bear Hill Road  
Waltham, MA 02451  
Attention: Stephen D. Lynch

With a copy to: Goulston & Storrs, P.C.  
400 Atlantic Avenue  
Boston, MA 02110  
Attention: Colleen P. Hussey, Esquire

If to Tenant: uniQure Inc. a Delaware corporation  
c/o Philip Astley-Sparke  
8 Amanda Lane  
Weston, MA 02493

With a copy to: DLA Piper LLP (US)  
33 Arch Street  
Boston, MA 02110

Notwithstanding the foregoing, any notice from Landlord to Tenant regarding ordinary business operations (e.g., exercise of a right of access to the Premises, maintenance activities, invoices, etc.) that do not have a binding legal effect may also be given by written notice delivered by facsimile to any person at the Premises whom Landlord reasonably believes is authorized to receive such notice on behalf of Tenant without copies as specified above. Either party may at any time change the address or specify an additional address for such Notices by delivering or mailing, as aforesaid, to the other party a notice stating the change and setting forth the changed or additional address, provided such changed or additional address is within the United States. Notices shall be effective (i.e., deemed received) upon the date of receipt or refusal thereof. Notice may be given by counsel to either party.

**25. GUARANTY.** Simultaneously with the execution of this Lease, Tenant shall deliver to Landlord an original guaranty in the form attached hereto as Exhibit 4 and incorporated herein (the "**Guaranty**.")

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executed by Guarantor as additional security for the payment and performance of certain of Tenant's obligations hereunder.

**26. MISCELLANEOUS**

**26.1 Separability.** If any provision of this Lease or portion of such provision or the application thereof to any person or circumstance is for any reason held invalid or unenforceable, the remainder of this Lease (or the remainder of such provision) and the application thereof to other persons or circumstances shall not be affected thereby.

**26.2 Captions.** The captions are inserted only as a matter of convenience and for reference, and in no way define, limit or describe the scope of this Lease nor the intent of any provisions thereof.

**26.3 Broker.** Tenant and Landlord each warrants and represents that it has dealt with no broker in connection with the consummation of this Lease other than Cushman & Wakefield ("**Broker**"). Tenant and Landlord each agrees to defend, indemnify and save the other harmless from and against any Claims arising in breach of the representation and warranty set forth in the immediately preceding sentence. Landlord shall be solely responsible for the payment of any brokerage commissions to Broker.

**26.4 Entire Agreement.** This Lease, Lease Summary Sheet and Exhibits 1-13 attached hereto and incorporated herein contain the entire and only agreement between the parties and any and all statements and representations, written and oral, including previous correspondence and agreements between the parties hereto, are merged herein. In the event of any conflict between any Exhibit and the body of this Lease (including without limitation the Lease Summary Sheet), the body of this Lease shall control. Tenant acknowledges that all representations and statements upon which it relied in executing this Lease are contained herein and that Tenant in no way relied upon any other statements or representations, written or oral. This Lease may not be modified orally or in any manner other than by written agreement signed by the parties hereto.

**26.5 Governing Law.** This Lease is made pursuant to, and shall be governed by, and construed in accordance with, the laws of the Commonwealth of Massachusetts and any applicable local municipal rules, regulations, by-laws, ordinances and the like.

**26.6 Representation of Authority.** By his or her execution hereof, each of the signatories on behalf of the respective parties hereby warrants and represents to the other that he or she is duly authorized to execute this Lease on behalf of such party. Upon Landlord's request, Tenant shall provide Landlord with reasonable evidence that any requisite resolution, corporate authority and any other necessary consents have been duly adopted and obtained.

**26.7 Expenses Incurred by Landlord Upon Tenant Requests.** Tenant shall, upon demand, reimburse Landlord for all reasonable third party expenses, including, without limitation, legal fees, incurred by Landlord in connection with the review and approval of Tenant's plans and specifications in connection with proposed Alterations to be made by Tenant to the Premises or in connection with requests by Tenant for Landlord's consent to make a Transfer or in connection with a request for a Landlord waiver pursuant to Section 8. Such costs shall be deemed to be additional rent under this Lease. Notwithstanding the foregoing, Tenant shall not be obligated to reimburse Landlord for third party expenses incurred in connection with the review and approval of Tenant's plans and specifications for Tenant's Work and charged to Landlord by Landlord's architect and/or engineers to the extent Tenant engages the same architect and/or engineers.

**26.8 Incentives.** Landlord shall reasonably cooperate with Tenant at no material cost and expense to Landlord in making application for forms of state and municipal financial assistance with

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respect to Tenant's relocation to the Building. To the extent Tenant provides Landlord with reasonable evidence that any such state or municipal financial assistance, including without limitation TIFA, is attributable to Tenant's occupancy of the Premises, then the whole of any economic benefit therefrom shall inure solely to Tenant.

**26.9 Survival.** Without limiting any other obligation of the parties which may survive the expiration or prior termination of the Term, all obligations on the part of Landlord or Tenant to indemnify, defend, or hold the other harmless, as set forth in this Lease shall survive the expiration or prior termination of the Term for three (3) years (or such other period as may be expressly set forth herein).

**26.10 Limitation of Liability.** Tenant shall neither assert nor seek to enforce any claim against Landlord or any of the Landlord Parties, or the assets of any of the Landlord Parties, for breach of this Lease or otherwise, other than against Landlord's interest in the Building and in the uncollected rents, issues, insurance and condemnation proceeds, and profits thereof, and Tenant agrees to look solely to such interest for the satisfaction of any liability of Landlord under this Lease. This Section 26.10 shall not limit any right that Tenant might otherwise have to obtain injunctive relief against Landlord. **Landlord and Tenant specifically agree that in no event shall any officer, director, trustee, employee or representative of Landlord or Tenant or any of the other Landlord Parties or any of the other Tenant Parties ever be personally liable for any obligation under this Lease, nor shall Landlord or any of the other Landlord Parties or Tenant or any of the other Tenant Parties be liable for consequential or incidental damages or for lost profits whatsoever in connection with this Lease (except pursuant to Sections 17 and/or 21.3).**

**26.11 Binding Effect.** The covenants, agreements, terms, provisions and conditions of this Lease shall bind and benefit the successors and assigns of the parties hereto with the same effect as if mentioned in each instance where a party hereto is named or referred to, except that no violation of the provisions of Section 13 hereof shall operate to vest any rights in any successor or assignee of Tenant.

**26.12 Landlord Obligations upon Transfer.** Upon any sale, transfer or other disposition of the Building, Landlord shall be entirely freed and relieved from the performance and observance thereafter of all covenants and obligations hereunder on the part of Landlord to be performed and observed to the extent that such covenants and obligations are assumed by Landlord's successor, it being understood and agreed in such event (and it shall be deemed and construed as a covenant running with the land) that the person succeeding to Landlord's ownership of said reversionary interest shall thereupon and thereafter assume, and perform and observe, any and all of such covenants and obligations of Landlord.

**26.13 No Grant of Interest.** Notwithstanding anything to the contrary contained herein, Tenant shall not grant any interest whatsoever in any fixtures within the Premises or any item paid in whole or in part by Landlord's Contribution or by Landlord.

**26.14 Prevailing Party.** If any party to this Agreement shall institute an action to enforce the terms hereof, the prevailing party shall be entitled to reasonable attorneys' fees and costs from the party determined to have breached the terms of this Agreement. An award of reasonable attorneys' fees and costs shall be determined by the court. The "prevailing party" shall be the party that obtains final judgment in its favor. If the institution of an action to enforce the terms of this contract is resolved by settlement, the parties will determine what portion, if any, of the injured party's legal fees and costs will be paid by the breaching party.

**26.15 Force Majeure.** Neither Landlord nor Tenant shall in any event be liable for failure to perform any of its obligations under this Lease when prevented from doing so by causes beyond its reasonable control, including, without limitation, labor disputes affecting the area generally, breakdown, accident, order or regulation of or by any governmental authority, or failure of supply, or inability by the

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exercise of reasonable diligence to obtain supplies, parts, or employees necessary to furnish services required under this Lease, or because of war or other emergency, (each, an event of "**Force Majeure**"); provided, however, in no event shall any such cause limit, affect, delay, detract from, or abate the obligations of either party to make any payments required under this Lease on a timely basis in accordance with the terms and conditions hereof.

**26.16 Confidentiality.**

(a) In connection with this Lease, from time to time Tenant has delivered and/or will deliver to Landlord, and the Landlord Parties may observe or have the opportunity to review, certain information about Tenant and/or its affiliates, including but not limited to financial information and other information related to the business operations of Tenant and/or its affiliates (such information whether furnished, observed, or reviewed before or after the Execution Date, whether oral, written, or visual, and regardless of the manner in which it is furnished, observed or reviewed, is collectively hereinafter referred to as "**Tenant's Proprietary Information**"). Tenant's Proprietary Information does not include, however, information which (1) is or becomes generally available to the public other than as a result of a disclosure in violation of this Section 26.16 by Landlord or Landlord's Engaged Persons (as defined below); (2) was available to Landlord on a non-confidential basis prior to its disclosure by Tenant; or (3) becomes available to Landlord on a non-confidential basis from a person other than Tenant who is not to the knowledge of Landlord or Landlord's Engaged Persons otherwise bound by a confidentiality agreement with Tenant, or is otherwise not under an obligation to Tenant not to transmit the information to Landlord.

(i) Landlord hereby covenants and agrees (A) to keep all Tenant's Proprietary Information confidential; (B) not to disclose or reveal any Tenant's Proprietary Information to any person other than those persons, including without limitation its and its affiliates' employees, investors, lenders, agents and representatives, whose duties and responsibilities reasonably require that Tenant's Proprietary Information be disclosed to them in connection with the ownership, financing, and/or sale of any of Landlord's interest in and to the Property or any portion thereof including the Premises (such persons are hereinafter referred to as "**Landlord's Engaged Persons**"); (C) to cause Landlord's Engaged Persons to observe the terms of this Section 26.16; and (D) not to use any Tenant's Proprietary Information for any purpose other than in connection with the ownership, financing, and/or sale of any of Landlord's interest in and to the Property or any portion thereof including the Premises.

(ii) In the event that Landlord is requested pursuant to, or required by, applicable law or regulation or by legal process to disclose any Tenant's Proprietary Information, Landlord agrees that it will provide Tenant with reasonable notice of such request or requirement in order to enable Tenant to seek an appropriate protective order or other remedy, to resist or narrow the scope of such request or legal process, or to waive compliance, in whole or in part, with the terms of this Section 26.16. In any such event Landlord will use reasonable efforts under the circumstances in which disclosure is sought to ensure that all Tenant's Proprietary Information will be accorded confidential treatment by the entity compelling such disclosure and Tenant shall respond in such a time and manner that does not put Landlord or any of its Engaged Persons at risk of violation of such law or regulation or legal process.

(iii) Without prejudice to the rights and remedies otherwise available at law or in equity, Landlord agrees that Tenant shall be entitled to seek equitable relief by way of injunction or otherwise if Landlord or any of Landlord's Engaged Persons breach or threaten to breach any of the provisions of this Section 26.16.

(b) In connection with this Lease, from time to time Landlord has delivered and/or will deliver to Tenant certain information about the Property, which may include, without limitation, title,

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zoning, geotechnical, permitting, environmental and operational materials relating to the Property (such information whether furnished, observed, or reviewed before or after the Execution Date, whether oral, written, or visual, and regardless of the manner in which it is furnished, observed or reviewed, is collectively hereinafter referred to as "**Landlord's Proprietary Information**"). Landlord's Proprietary Information does not include, however, information which (1) is or becomes generally available to the public other than as a result of a disclosure in violation of this Section 26.16 by Tenant or Tenant's Engaged Persons (as defined below); (2) was available to Tenant on a non-confidential basis prior to its disclosure by Landlord; or (3) becomes available to Tenant on a non-confidential basis from a person other than Landlord who is not to the knowledge of Tenant or Tenant's Engaged Persons otherwise bound by a confidentiality agreement with Landlord, or is otherwise not under an obligation to Landlord not to transmit the information to Tenant.

(i) Tenant hereby covenants and agrees (A) to keep all Landlord’s Proprietary Information confidential; (B) not to disclose or reveal any Landlord’s Proprietary Information to any person other than those persons, including without limitation its and its affiliates’ employees, investors, lenders, agents and representatives, whose duties and responsibilities reasonably require that Landlord’s Proprietary Information be disclosed to them in connection with this Lease (such persons are hereinafter referred to as “**Tenant’s Engaged Persons**”); (C) to cause Tenant’s Engaged Persons to observe the terms of this Section 26.16; and (D) not to use any Landlord’s Proprietary Information for any purpose other than in connection with this Lease.

(ii) In the event that Tenant is requested pursuant to, or required by, applicable law or regulation or by legal process to disclose any Landlord’s Proprietary Information, Tenant agrees that it will provide Landlord with reasonable notice of such request or requirement in order to enable Landlord to seek an appropriate protective order or other remedy, to resist or narrow the scope of such request or legal process, or to waive compliance, in whole or in part, with the terms of this Section 26.16. In any such event Tenant will use reasonable efforts under the circumstances in which disclosure is sought to ensure that all Landlord’s Proprietary Information will be accorded confidential treatment by the entity compelling such disclosure and Landlord shall respond in such a time and manner that does not put Tenant or any of its Engaged Persons at risk of violation of such law or regulation or legal process.

(iii) Without prejudice to the rights and remedies otherwise available at law or in equity, Tenant agrees that Landlord shall be entitled to seek equitable relief by way of injunction or otherwise if Tenant or any of Tenant’s Engaged Persons breach or threaten to breach any of the provisions of this Section 26.16.

(c) Landlord will be responsible for any breach of the terms of this Section 26.16 by it and/or Landlord’s Engaged Persons. Tenant will be responsible for any breach of the terms of this Section 26.16 by it and/or Tenant’s Engaged Persons.

(d) The obligations of the parties under this Section 26.16 shall survive the expiration or prior termination of the Term for three (3) years.

**26.17 Financial Information.** Unless Tenant is traded on a U.S. public stock exchange, Tenant shall deliver to Landlord, within thirty (30) days after Landlord’s reasonable request (which request may be made no more than two (2) times per year, provided that such limitation shall not apply in the event of a sale or financing of any of Landlord’s interest in the Lease or the property of which the Premises are a part, or if there is an Event of Default by Tenant under this Lease), (a) Tenant’s most recently completed audited statements of income, shareholder’s equity and cash flows statements, and (b) Tenant’s most recently completed balance sheet (audited or reviewed by an independent certified public accountant if available, and certified by an officer of Tenant, if not so reviewed or audited, as being true

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and correct in all material respects). Any such financial information may be relied upon by any actual or potential lessor, purchaser, or mortgagee of the Property or any portion thereof.

[SIGNATURES ON FOLLOWING PAGE]

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IN WITNESS WHEREOF the parties hereto have executed this Lease as a sealed instrument as of the Execution Date.

**LANDLORD**

KING 113 HARTWELL LLC

By: King Dickey LLC, its manager

By: King Street Properties Investments LLC, its manager

By: \_\_\_\_\_

Name:

Title:

**TENANT**

UNIQUE, INC.

By: /s/PJ Morgan \_\_\_\_\_

Name: PJ Morgan

Title: CFO Treasurer

By: /s/Jörn Aldag \_\_\_\_\_

Name: Jörn Aldag

Title: CEO

Signature Page

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EXHIBIT 1A

LEASE PLAN

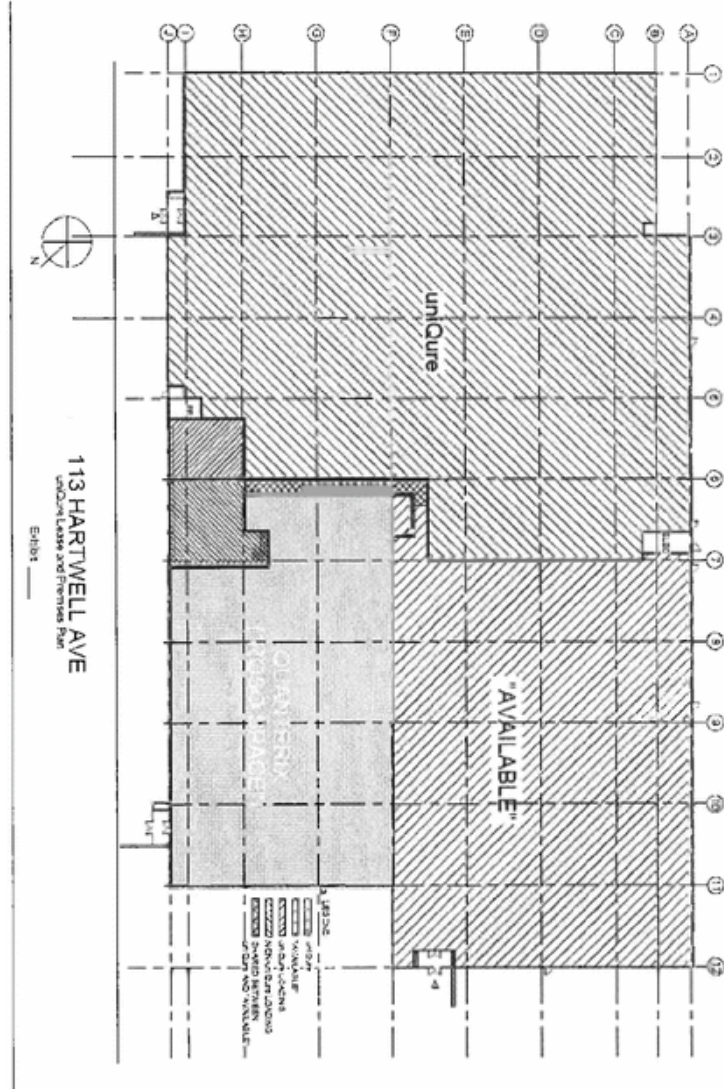


EXHIBIT 1A, PAGE 1

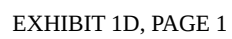
EXHIBIT 1B

PLAN OF TENANT'S EXCLUSIVE PARKING SPACES AND COMMON PARKING AREA









PLAN OF OUTDOOR PATIO LOCATION



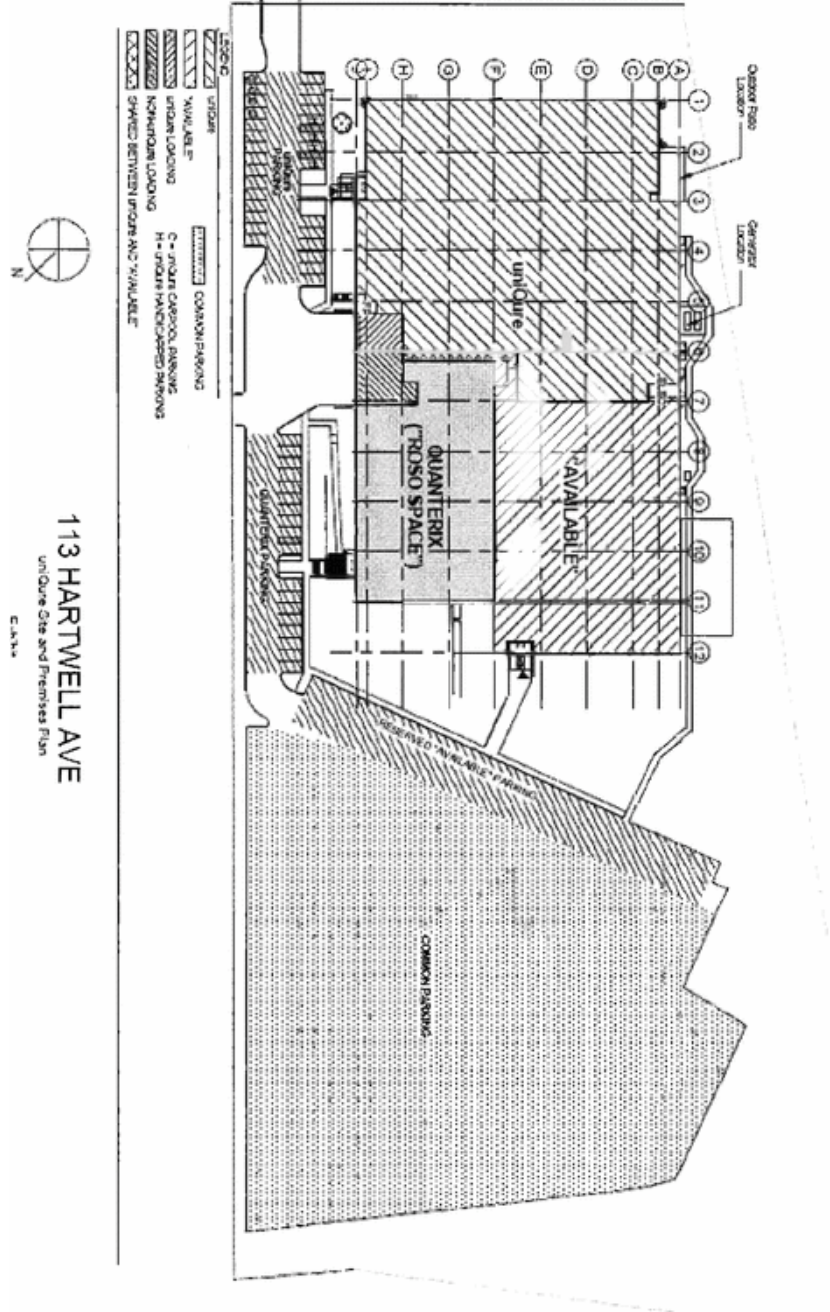


EXHIBIT 1E, PAGE 1

EXHIBIT 1F

PLAN OF ROSO SPACE

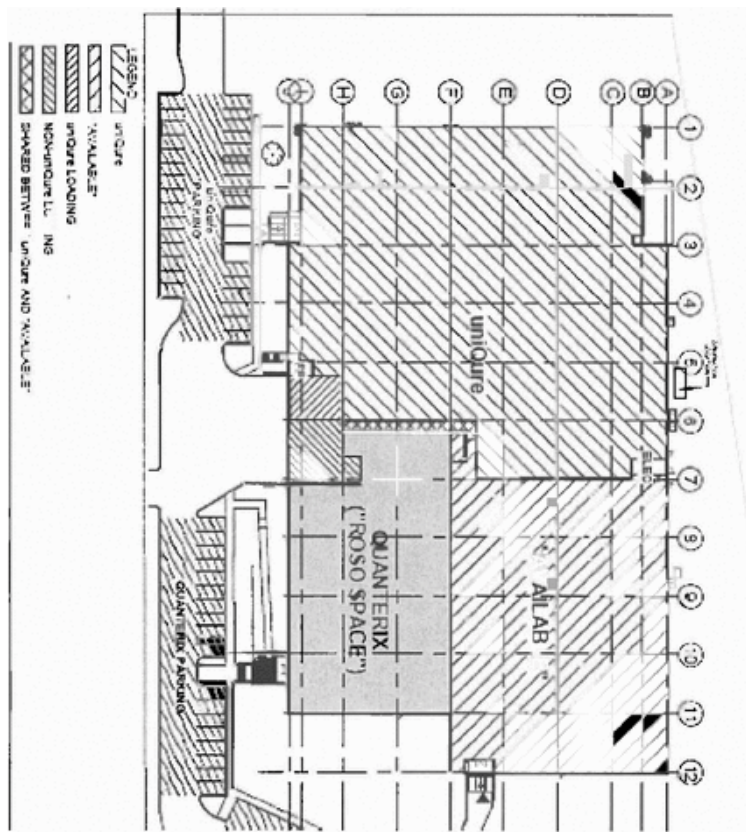


EXHIBIT 1F, PAGE 1

EXHIBIT 1G

PLAN OF UNLEASED SPACE

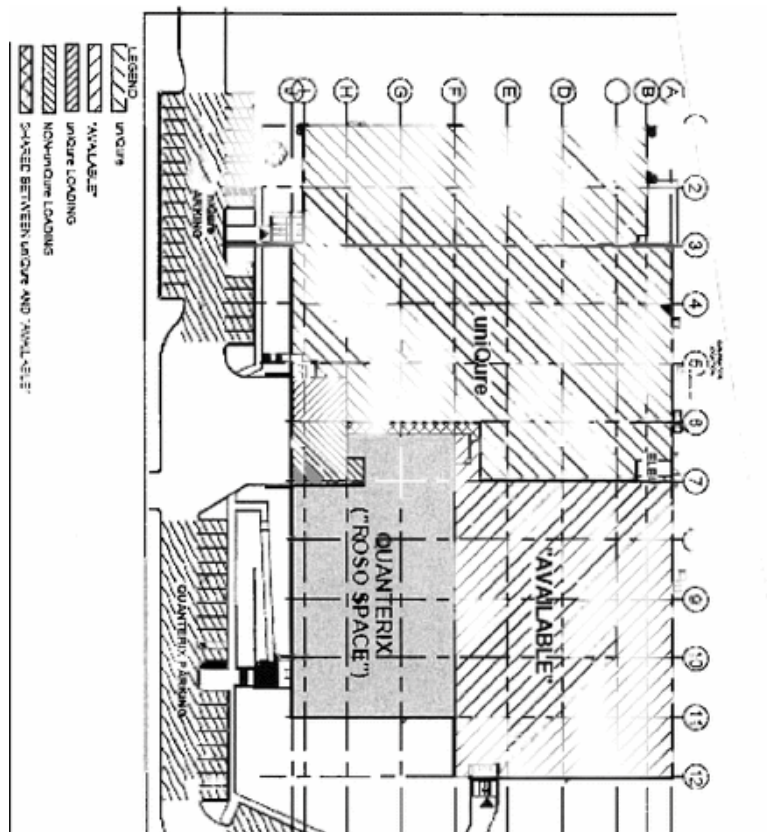


EXHIBIT 1G, PAGE 1

EXHIBIT 2

LEGAL DESCRIPTION

That certain parcel of land with the buildings and improvements located thereon situate in Lexington in the County Middlesex and Commonwealth of Massachusetts, described as follows:

SOUTHEASTERLY by the northwesterly line of Hartwell Avenue, three hundred fifty-four and 10/100 feet;

SOUTHERLY by lot 6 as shown on plan hereinafter mentioned, ten hundred fifty-one and 58/100 feet;

NORTHWESTERLY by land now or formerly of The United States of America, five hundred eighteen and 89/100 feet; and

NORTHEASTERLY by lot 4 on said plan, nine hundred ninety-one and 79/100 (79/100) feet.

Said parcel is shown as Lot 5 on said plan (Plan No. 31330B)

All of said boundaries are determined by the Court to be located as shown on a subdivision plan, as approved by the Court, filed in the Land Registration Office, a copy of which is filed in the Registry of Deeds for the South Registry District of Middlesex County in Registration Book 756, Page 132, with Certificate 125282.

The above described land has the benefit of the ditches as approximately shown on Plan filed in Registration Book 685, Page 171, at date of original decree (May 17, 1963).

EXHIBIT 2, PAGE 1

EXHIBIT 3A

MATRIX

113 HARTWELL AVE  
Exhibit 3 Landlord's Work  
May 29, 2013

Category	Landlord	Tenant
Exterior sitework to include realigned entrance from Hartwell Ave, curbing and parking at Tenant entrance, final paving and striping of driveway, sealcoat and stripe main parking area, finish landscaping and sidewalks.*	X	
New main entrance comprised of extenor metal panel, glass storefront, illuminated monumental fin wall (ready to receive Tenant signage), steel and glass canopy. Vestibule doors and vestibule in painted drywall and drywall ceiling, finished with file border and carpet inset. Skylight and clerestory glass (above stair).	X	
Handrail at stair located near main entry and stair finishes.		X
On southern façade generally between column lines 3 and 7 on the Premises Plan new EFIS extenor with new Windows with insulated low E glass. Facade and windows in all other locations comprising Premises will be as-is.	X	
Two (2) full height (48") loading docks (one with dock leveler) with overhead doors located within five (5) bay common loading dock.	X	
New full height demising wall with 5/8" GWB, 3 5/8" on studs, 16" on center, fire taped and sealed to the deck on vacant side only (open framing on Tenant facing side).	X	
Interior demolition of offices, bathroom and shed. Premises to be in broom clean, as-is condition including interior face of exterior walls except between column lines 3 and 7.	X	
Raise 8 bays of roof ("High Bay Area") to height of 24' clear to underside of joists. Includes demolition, new structure, roof drains, new TPO roof and new metal panel on walls.	X	
New TPO roof with 15 Year Warranty	X	
Install on new 1600 Amp - 480V 3Ph service and one new 400 Amp - 480 V 3Ph service to the location of Tenant electrical closet with distribution panel and disconnect switch for each of the 2 services.	X	
Sprinkler: Mains and branch lines in a grid per code with heads turned up. In the High Bay Area mains and branch lines will be removed and capped at the perimeter of the High Bay Area.		X
Install 3" natural gas line stubbed and capped at the demising wall	X	
Install 2" domestic water service with backflow preventer stubbed to Premises.	X	
Install (2) fire alarm modules to Premises.	X	
Fire alarm programming by Tenant using Landlord's fire alarm contractor		X
Ph pit and 6" Acid waste outlet to be connected to Town sewer, outside of building footprint in new manhole.		X
New underground sewer line tied to Town sewer.		X
Security system, card access system, alarm system and teldata.		X

Exterior patio on south elevation.	X
Any dunnage and associated roofing for equipment. Note: Tenant must use Landlord’s roofing contractor.	X
HVAC	X
Bathrooms: in accordance with all applicable codes and Landlord’s standards.	X

EXHIBIT 3B

LANDLORD WORK PLANS

ARCHITECTURAL DRAWING LIST

- COVER SHEET
- A0.3 SITE PLAN
- A0.4 FIRST FLOOR DEMO PLAN
- A1.0 FIRST FLOOR PLAN
- A2.0 REFLECTED CEILING PLAN
- A1.1 ROOF PLAN- NEW
- A3.0 EXTERIOR ELEVATION
- A3.1 EXTERIOR ELEVATIONS
- A4.0 ELEVATOR AND STAIR SECTIONS
- A5.0 TYPICAL WALL SECTIONS AND DETAILS
- A5.1 S.E. WALL SECTIONS AND DETAILS
- A5.2 EAST FACADE IMPROVEMENTS
- A5.3 REAR FACADE IMPROVEMENTS
- A5.4 RAIDE ROOF SECTIONS AND DETAILS
- A6.0 LOADING DOCK SECTIONS
- A6.1 ATRIUM ELEVATIONS
- A6.2 ENTRY #2 SECTION AND DETAILS
- a6.3 ENTRY #3 SECTION AND DETAILS
- A6.4 ROOF DETAILS
- A7.0 ENLARGED PLANS
- A7.1 ENLARGED PLANS
- A9.0 DOOR, PANEL, AND STROREFRONT TYPES, AND DOOR SCHEDULE

STRUCTURAL DRAWING LIST

- S0.01 GENERAL NOTES
- S0.02 TYPICAL DETAILS
- S1.01 FIRST FLOOR PLAN
- S1.02 ROOF PLAN
- S1.03 HIGH ROOF PLAN
- S2.01 SECTIONS & DETAILS
- S2.03 SECTIONS & DETAILS
- S2.04 SECTIONS & DETAILS
- S2.06 SECTIONS & DETAILS
- S2.07 BRACING ELEVATIONS & DETAILS
- S3.01 SECTIONS & DETAILS
- S7.01 SECTIONS & DETAILS

EXHIBIT 3C

TENANT’S PROGRAM





**Owner**

uniGuns, Inc.  
P.O. Box 22508  
CA, Amsterdam

Tel

Fax

Mr. Steve Gorgol

Tel

Cell

E-mail: [stevehorgol@comcast.net](mailto:stevehorgol@comcast.net)

**General Contractor**

The Richmond Group, Inc.  
77 Main Street  
Malden, MA 02148

Tel: (508) 495-0700

Fax: (508) 495-0716

Mr. Amy Foley

Project Planner

Tel: (508) 495-0700

Cell: (508) 328-1895

E-mail: [afoley@therichmondgroup.org](mailto:afoley@therichmondgroup.org)

Mr. Richard Lefebvre

Executive Vice President

Tel: (508) 495-0700

Cell: (508) 958-0024

E-mail: [rlfebvre@therichmondgroup.org](mailto:rlfebvre@therichmondgroup.org)

**Engineer**

Goldstein-Milano, LLC  
125 Main Street  
Reading, MA 01867

**Structural**

Tel: (781) 670-0000

Fax: (781) 670-0039

Mr. Christopher Milano

Principal

Tel: (781) 670-0000

Cell: (781) 439-2208

E-mail: [cpm@gmllc.com](mailto:cpm@gmllc.com)

**Consultant**

Rolf Jensen & Associates, Inc.  
1881 Worcester Road  
Suite 501  
Framingham, MA 01701

**Building Code**

Tel: (508) 620-8000

Fax: (508) 620-0008

Mr. Chris Lynch

Fire Protection Consultant

Tel: (508) 620-8000

Cell: N/A

E-mail: [clynch@rja-group.com](mailto:clynch@rja-group.com)

**Subcontractor**

Environmental Systems Inc.

**HVAC**

<b>Subcontractor</b>		
<b>Edward Island Drive</b> Adelphi, MA 02723	Tel: (508) 228-8008	Fax: (508) 222-1944
Mr. Jim Barlow E-mail: jbarlow@edwardsma.net	Tel: (508) 228-8008	Cell: (401) 524-9193
Mr. Robert Kelley, Jr. E-mail: rkelly@edwardsma.net	Tel: (508) 228-8008	Cell: (508) 294-8481
<b>Interstate Electrical Services Corp.</b>		
20 Trilene Cove Road N. Attleboro, MA 01962	<b>Electrical</b> Tel: (978) 667-5200	Fax: (978) 947-8259
Mr. Chris McAuley E-mail: cmcauley@interstate1.com	Tel: (978) 667-5200	Cell: (978) 560-5453
<b>Norelco Sprinkler Corporation</b>		
150 Perry Street East Douglas, MA 01918	<b>Fire Protection</b> Tel: (508) 478-1037	Fax: (508) 478-9156
Mr. Wayne Davis E-mail: wayne@norelcosprinkler.com	Tel: (508) 478-1037	Cell: (508) 400-2048
<b>North Shore Mechanical Contractors</b>		
6 Garden Street, Suite 2 Dorchester, MA 01923	<b>Plumbing</b> Tel: (978) 774-6600	Fax: (978) 887-5002
Mr. Joe Whitney E-mail: joe_whitney@nsmech.com	Tel: (978) 774-6600	Cell: (978) 423-6467
<b>Architect &amp; Engineer</b>		
<b>Industrial Facilities Design, Inc.</b>	<b>Facilities Planning &amp; Management</b>	
85 Main Street Mendon, MA 01752	Tel: (508) 544-1895	Fax: (508) 544-1894
Mr. George Davis E-mail: ggd@ifd.net	Tel: (508) 544-1895	Cell: N/A
Mr. Rob Tricock E-mail: rtricock@ifd.net	Tel: (508) 544-1895	Cell: (508) 940-5637
<b>Architects</b>		
<b>Dimella Shaffer Associates</b>	<b>Architecture &amp; Planning</b>	
289 Congress Street Boston, MA 02210	Tel: (617) 428-5004	Fax: (617) 428-0048
Mr. David Gaudin E-mail: DGaudin@dimellashaffer.com	Tel: (617) 428-5004	Cell: (617) 428-5004
Ms. Jessica Schulte E-mail: jschulte@dimellashaffer.com	Tel: (617) 428-5004	Cell:

Printed on: 8/17/2012

Page 2

EXHIBIT 3D, PAGE 2

<b>Architects</b>		
<b>Engineering</b>		
<b>Kevin Gregory Engineering</b>	<b>Mechanical</b>	
109 Franklin Street Stoughton, MA 02159	Tel: (781) 885-0185	Fax: (781) 885-3072
Mr. Kevin Gregory E-mail: kgregory@kge-inc.com	Tel: (781) 885-0185	Cell: (781) 820-4472
<b>Vendor</b>		
<b>Quality Systems Integration, LLC.</b>	Tel:	Fax:
Mr. Shaun McCormick E-mail: shaun@qsinc.com	Tel:	Cell:

EXHIBIT 3D, PAGE 3

EXHIBIT 3E

LANDLORD'S WORK CONSTRUCTION SCHEDULE

The dates set forth in this schedule were predicated on the Execution Date occurring prior to June 15, 2013. The dates set forth herein are not binding in any manner, and in no event shall any of the dates set forth in such schedule be deemed to amend or modify Landlord's obligations or Tenant's rights set forth in Section 3 of the Lease.

KING STREET PROPERTIES BASE BUILDING & UNDORE ROOF									
Task Name	Duration	Start	Finish	May	June	July	August	September	October
1				1	2	3	4	5	6
2				7	8	9	10	11	12
3				13	14	15	16	17	18
4				19	20	21	22	23	24
5				25	26	27	28	29	30
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35									
B.W. KENNEDY & CO.									
1 of 4									



KING STREET PROPERTIES  
BASE BUILDING & UNIQUE ROOF

ID	Task Name	Duration	Start	Finish	May B.M.E	June B.M.E	July B.M.E	August B.M.E	September B.M.E	October B.M.E	November B.M.E
36	Exterior concrete & rebar	7 days	Tue 7/23/13	Wed 7/23/13							
37	Metal panel	10 days	Wed 7/23/13	Tue 7/23/13							
38	Stonefront	10 days	Wed 7/23/13	Tue 7/23/13							
39	Elevator	15 days	Wed 7/23/13	Tue 7/30/13							
40	Landscape	10 days	Tue 7/23/13	Mon 8/5/13							
41	Plumbing	10 days	Wed 7/23/13	Tue 7/30/13							
42	HVAC	10 days	Wed 7/23/13	Tue 7/30/13							
43	Building electrical	10 days	Wed 7/23/13	Tue 7/23/13							
44	Exterior electrical	10 days	Tue 7/23/13	Mon 8/5/13							
45	Procurement	81 days	Tue 7/23/13	Thu 10/3/13							
46	Joists	25 days	Wed 7/23/13	Tue 8/20/13							
47	Structural steel	25 days	Wed 7/23/13	Tue 8/20/13							
48	Roofing	3 days	Tue 7/23/13	Thu 7/25/13							
49	Building rest	5 days	Fri 7/19/13	Thu 7/25/13							
50	Site work	5 days	Thu 8/1/13	Wed 8/7/13							
51	Exterior rest	5 days	Thu 8/1/13	Wed 8/7/13							
52	Framing	5 days	Wed 7/23/13	Tue 7/23/13							
53	Metal panel	15 days	Wed 7/23/13	Tue 8/13/13							
54	Windows	20 days	Wed 7/23/13	Tue 8/20/13							
55	Stonefront	50 days	Wed 7/23/13	Wed 10/2/13							
56	Elevator	65 days	Wed 7/23/13	Thu 10/3/13							
57	Landscape	10 days	Tue 8/6/13	Mon 8/19/13							
58	Plumbing	5 days	Wed 7/23/13	Tue 8/6/13							
59	HVAC	30 days	Wed 7/23/13	Wed 8/13/13							
60	Building electrical	30 days	Wed 7/23/13	Wed 8/13/13							
61	Exterior electrical	30 days	Tue 8/6/13	Tue 8/13/13							
62	Permitting	56 days	Mon 6/24/13	Wed 8/14/13							
63	Building permit	23 days	Tue 7/23/13	Thu 8/1/13							
64	National Grid gas service	50 days	Tue 7/23/13	Wed 9/11/13							
65	FAA permit	45 days	Mon 8/24/13	Mon 8/28/13							
66	Site plan approval	30 days	Tue 7/23/13	Mon 8/19/13							
67	Tennessee Gas review	15 days	Tue 7/23/13	Mon 7/29/13							
68											
69	CONSTRUCTION	92 days	Fri 7/25/13	Wed 11/13/13							
70	Entry 1	72 days	Fri 7/25/13	Wed 10/16/13							

Tue 8/25/13

B.W. KENNEDY & CO.

ARCHITECT & ENGINEERING ARCHITECTS

ID	Task Name	Duration	Start	Finish	May	June	July	August	September	October	November
71	Construction fence	1 day	Fri 7/5/13	Fri 7/5/13							
72	Make safe	2 days	Mon 7/8/13	Tue 7/9/13							
73	Reclaim from low roof m/s	1 day	Mon 7/8/13	Mon 7/8/13							
74	Strip roof @ atrium & office	2 days	Tue 7/9/13	Wed 7/10/13							
75	New roof over office	1 day	Fri 7/12/13	Fri 7/12/13							
76	Shoring and demo	6 days	Fri 7/12/13	Fri 7/19/13							
77	Strip balance of low roof	3 days	Mon 7/15/13	Wed 7/17/13							
78	Install new roof up to atrium	5 days	Thu 7/18/13	Wed 7/24/13							
79	Excavate for foundation	3 days	Mon 7/22/13	Wed 7/24/13							
80	Foundation for building	10 days	Thu 7/25/13	Wed 8/7/13							
81	Foundation for elevator	5 days	Wed 7/31/13	Tue 8/6/13							
82	Backfill	2 days	Thu 8/8/13	Fri 8/9/13							
83	CMU for elevator	5 days	Wed 8/14/13	Tue 8/20/13							
84	1-stall steel	8 days	Wed 8/25/13	Mon 9/9/13							
85	Steel inspection	1 day	Tue 9/10/13	Tue 9/10/13							
86	Interior slabs	5 days	Tue 9/17/13	Mon 9/23/13							
87	Framing	5 days	Wed 9/18/13	Tue 9/17/13							
88	Fish bottom of walls	2 days	Mon 9/16/13	Tue 9/17/13							
89	Metal panel	5 days	Wed 9/18/13	Tue 9/24/13							
90	Windows & storefront	5 days	Wed 9/25/13	Tue 10/1/13							
91	Install roof adjacent & above atrium	4 days	Fri 9/27/13	Wed 10/2/13							
92	Roof coping	2 days	Thu 10/3/13	Fri 10/4/13							
93	Canopy steel, framing, roof & glass	7 days	Wed 10/2/13	Thu 10/10/13							
94	Vestibule finishes	9 days	Thu 10/3/13	Wed 10/16/13							
95	Existing and Raised Roof	70 days	Tue 7/9/13	Wed 10/16/13							
96	Relocate existing utilities	8 days	Wed 7/10/13	Fri 7/19/13							
97	Reclaim floor from m/s	3 days	Tue 7/9/13	Thu 7/11/13							
98	Demo existing roof top units	3 days	Mon 7/22/13	Wed 7/24/13							
99	Strip existing roof	15 days	Thu 7/25/13	Wed 8/14/13							
100	1-stall roof 7 to 12 lines	10 days	Thu 8/15/13	Wed 8/28/13							
101	Relocate joists	5 days	Wed 8/27/13	Tue 9/3/13							
102	1-stall steel @ raised section	8 days	Tue 9/3/13	Thu 9/12/13							
103	Steel inspection	1 day	Fri 9/13/13	Fri 9/13/13							
104	Framing	6 days	Mon 9/16/13	Mon 9/23/13							
105	Fish bottom of walls	2 days	Fri 9/20/13	Mon 9/23/13							

TABLE 1. CONTINUED

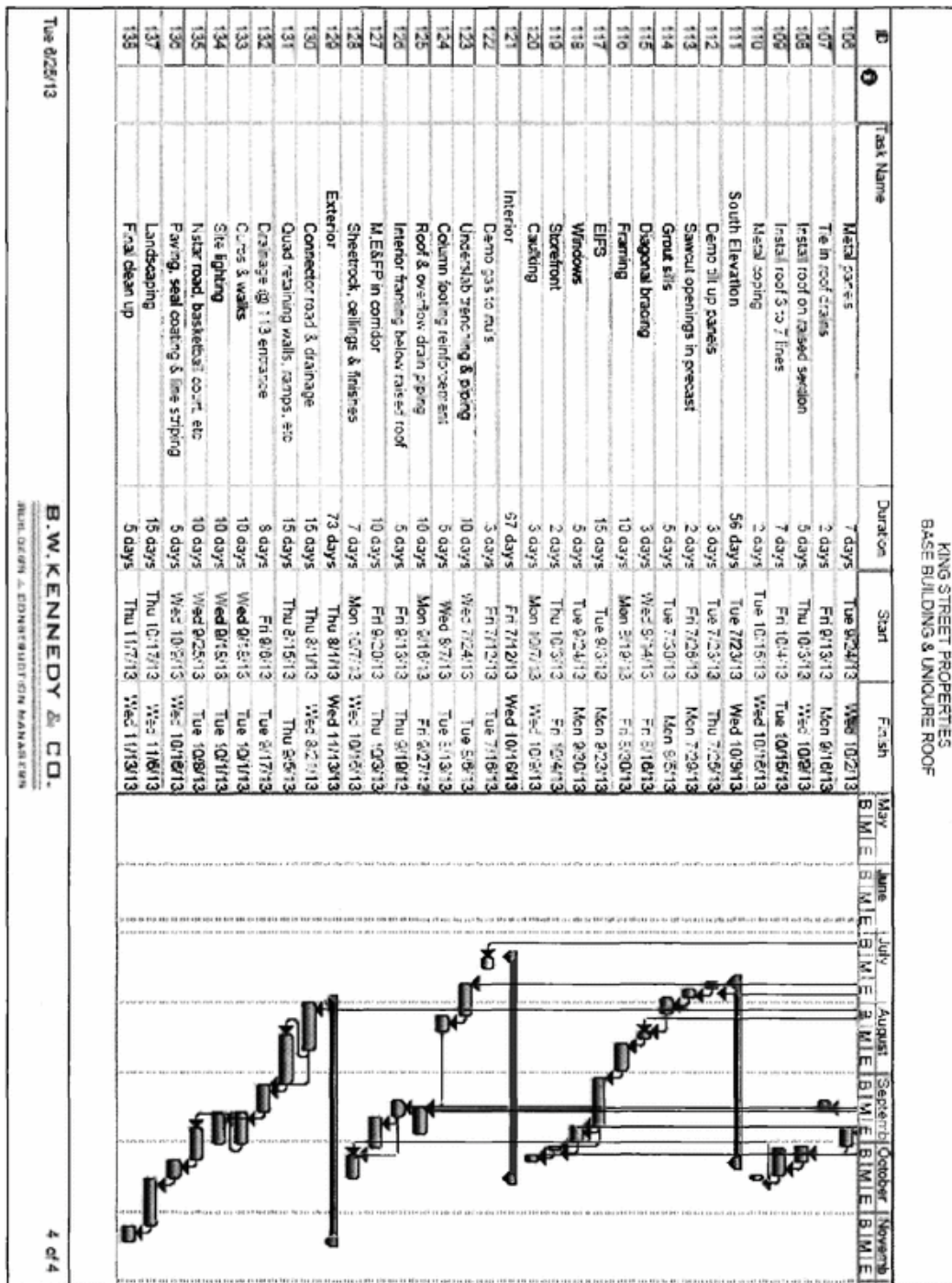


EXHIBIT 3E, PAGE 4

## EXHIBIT 4

## FORM OF GUARANTY

### GUARANTY

This GUARANTY dated as of July , 2013 is made by uniQure BV, a private limited liability company organized under the laws of the Netherlands (the “**Guarantor**”), in favor of King 113 Hartwell LLC, a Massachusetts limited liability company (“**Landlord**”).

uniQure, Inc., a Delaware corporation (“**uniQure**”) and Landlord are parties to that certain Lease dated on or about the date hereof, as the same may be amended from time to time (collectively, the “**Lease**”) with respect to certain premises within the building located at 113 Hartwell Avenue, Lexington, Massachusetts (the “**Premises**”). In order to induce Landlord to enter into the Lease with uniQure, Guarantor has agreed to execute and deliver this Guaranty to Landlord. For good and valuable consideration, the receipt and sufficiency of which are hereby acknowledged, Guarantor hereby agrees as follows:

1. **Guaranty of Payment.** The Guarantor hereby unconditionally guarantees the due and punctual payment of Base Rent, and all other additional rent, interest and charges due from uniQure under the Lease (the “**Guaranteed Obligations**”). Upon any failure by uniQure to pay any of the Guaranteed Obligations, the Guarantor agrees that it will forthwith on demand pay such amounts which uniQure has failed to pay. In no event shall Guarantor’s liability under this Guaranty exceed the sum of (a) the total amount of rent due with respect to the then-remaining term of the Lease, as it may be extended from time to time, (b) Landlord’s expenses, including reasonable attorneys’ fees and disbursements, incurred by Landlord in enforcing the obligations of uniQure under the Lease and/or the obligations of Guarantor under this Guaranty, and (c) such additional amount of rents that may be due on account of uniQure’s failure to vacate the Premises in the condition required upon the expiration or earlier termination of the Lease. All payments required to be made by Guarantor hereunder shall be paid to Landlord in legal United States currency or tender at Landlord’s address set forth below, or at such other address as Landlord may specify from time to time. This Guaranty is irrevocable, absolute, present, continuing and unconditional, and the obligations of Guarantor shall not be released, impaired, modified, limited or affected in any way by (a) any assignment or other transfer of the Lease or this Guaranty by Landlord; (b) any assignment or other transfer of the Lease by uniQure or the



sublease of all or part of the Premises by uniQure; (c) the release or discharge of uniQure in bankruptcy or other creditors' proceeding; or (d) any rejection or disclaimer of uniQure. In addition, the obligations hereunder of Guarantor shall extend and apply with respect to the full and faithful performance and observance of all Guaranteed Obligations (i) if the Lease shall be renewed, or its term extended, for any period beyond the date specified in the Lease for the expiration of said term, either pursuant to any option granted under the Lease or otherwise; and (ii) if uniQure holds over beyond the term of the Lease.

2. Discharge Only Upon Payment in Full; Reinstatement in Certain Circumstances. The Guarantor's obligations hereunder constitute a guarantee of payment and not of collection merely and shall remain in full force and effect until the Guaranteed Obligations have been paid in full. If at any time any payment of any of the Guaranteed Obligations is rescinded or must be otherwise restored or returned upon the insolvency, bankruptcy or reorganization of uniQure or otherwise, Guarantor's obligations hereunder with respect to such payment shall be reinstated at such time as though such payment has not been made.

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EXHIBIT 4, PAGE 1

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3. Waiver by the Guarantor. The Guarantor irrevocably waives acceptance hereof, diligence, presentment, demand, protest, notice of dishonor and any notice not provided for herein, as well as any requirement that at any time any person exhaust any right or take any actions against uniQure or its assets or any other guarantor or person.

4. Subrogation. Upon making any payment hereunder, the Guarantor shall be subrogated to the rights of Landlord against uniQure with respect to such payment; provided that the Guarantor shall not enforce any right or receive any payment by way of subrogation until all of the Guaranteed Obligations shall have been paid in full.

5. Assignment; Successors and Assigns; Termination. The Guaranty shall be binding upon and inure to the benefit of the Guarantor and its successors and assigns and Landlord and its successors and assigns. Guarantor may not assign its rights or its obligations hereunder without the prior written consent of Landlord, and any such purported assignment without the written consent of Landlord will be void.

6. Notices. Notices given under this Guaranty shall be in writing and delivered, sent or transmitted in the manner set forth in the Lease (i) if to Landlord, to it King 113 Hartwell LLC, c/o King Street Properties, 255 Bear Hill Road, Waltham, MA 02451, Attention: Stephen D. Lynch, with a copy to Goulston & Storrs, P.C., 400 Atlantic Avenue, Boston, MA 02110, Attention: Colleen P. Hussey, Esquire, and (ii) if to Guarantor, to it at: P.O. Box 22506, 1100 DA Amsterdam, The Netherlands, Attn: Mr. Piers J. Morgan, with a copy to DLA Piper LLP (US), 33 Arch Street, 26th Floor, Boston, Massachusetts 02110-1447, Attention: Geoff Howell, or such other address as Landlord or Guarantor has specified by written notice to the other. Except as otherwise specifically required herein, notice of the exercise of any right, option or power granted to Landlord by this Guaranty is not required to be given.

7. Amendments and Waivers. No provisions of this Guaranty may be amended, supplemented or modified, nor any of the terms and conditions hereof waived, except by a written instrument executed by the Guarantor and Landlord.

8. Certification of Status. Guarantor shall at any time and from time to time upon not less than ten (10) business days' prior notice from Landlord, execute, acknowledge and deliver to Landlord a statement in writing certifying that this Guaranty is unmodified and in full force and effect (or if there have been modifications, that the same is in full force and effect as modified and stating the modifications) it being intended that any such statement delivered pursuant hereto may be relied upon by any prospective purchaser of the Building or of any interest of Landlord therein, any Mortgagee or prospective Mortgagee thereof, any lessor or prospective lessor thereof, any lessee or prospective lessee thereof, or any prospective assignee of any mortgage thereof. If Guarantor fails to execute, acknowledge and deliver such a statement within such 10-day period, Landlord may send a notice to Guarantor thereof (which notice shall be delivered in an envelope that conspicuously states the following in bold caps: "**DEFAULT BY GUARANTOR WITH RESPECT TO LEXINGTON, MA LEASE**" and which notice shall include an explicit statement that such notice is a notice delivered pursuant to this Section 8 and Guarantor's failure to perform the specified obligation will trigger the provisions of this Section 8). If Guarantor fails to execute, acknowledge and deliver such a statement within five (5) business days after receipt of such a notice from Landlord, such failure shall constitute a material default by Guarantor hereunder.

9. Report of Guarantor's Financial Position. Unless Guarantor is traded on a U.S. public stock exchange or other public stock exchange providing financial information in English readily available in the Boston area (whether via the internet or otherwise), Guarantor shall deliver to Landlord, within thirty (30) days after Landlord's reasonable request (which request may be made no more than two (2) times per year, provided that such limitation shall not apply in the event of a sale or financing of any

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of Landlord's interest in the Lease or the property of which the Premises are a part, or if there is an Event of Default by uniQure under the Lease), (a) Guarantor's most recently completed audited statements of income, shareholder's equity and cash flows statements, and (b) Guarantor's most recently completed balance sheet (audited or reviewed by an independent certified public accountant if available, and certified by an officer of Guarantor, if not so reviewed or audited, as presenting fairly, in all material respects, the financial position of Guarantor as of the date(s) thereof). Any such financial information may be relied upon by Landlord and any actual or potential lessor, purchaser, or mortgagee of the Property or any portion thereof. If Guarantor fails to deliver such information within such 30-day period, Landlord may send a notice to Guarantor thereof (which notice shall be delivered in an envelope that conspicuously states the following in bold caps: "**DEFAULT BY GUARANTOR WITH RESPECT TO LEXINGTON, MA LEASE**" and which notice shall include an explicit statement that such notice is a notice delivered pursuant to this Section 9 and Guarantor's failure to perform the specified obligation will trigger the provisions of this Section 9). If Guarantor fails to deliver such information within five (5) business days after receipt of such a notice from Landlord, such failure shall constitute a material default by Guarantor hereunder.

10. Miscellaneous.

a) Guarantor expressly agrees that the validity of this Guaranty and the obligations of Guarantor under this Guaranty shall not be terminated or in any way affected or impaired by reason of any amendment to the Lease, but shall continue in full force and effect with respect to the Lease as the Lease may be amended from time to time. Guarantor further expressly agrees that the validity of this Guaranty and the obligations of Guarantor under this Guaranty shall not be terminated or in any way affected or impaired by reason of the assertion by Landlord against uniQure of any of the rights or remedies reserved to Landlord pursuant to the provisions of the Lease, or by reason of the waiver or failure by Landlord to enforce any of the terms, covenants or conditions of the Lease, this Guaranty, or any other guaranty of the Lease (if any), or by reason of the granting of any indulgence or extension to uniQure, or Guarantor, or to any other guarantor (if any), all of which may be given or done by Landlord from time to time without notice to Guarantor. Guarantor waives notice of non-payment of rent, additional charges, or any other amounts to be paid by uniQure under the Lease, and waives notice of default or non-performance of any of uniQure's other covenants, conditions and agreements contained in the Lease. Guarantor further waives, to the fullest extent permitted by law, any and all legal,

equitable and/or surety defenses whatsoever to which Guarantor might otherwise be entitled other than: (1) that Guarantor has fully performed all of its obligations under this Guaranty, and (2) that uniQure has fully performed all of its obligations under the Lease (determined without regard to any relief of uniQure from its obligations by operation of law or otherwise).

b) Guarantor agrees that its liability under this Guaranty shall be primary and joint and several with uniQure, any other guarantor (if any), and any other party liable for uniQure’s obligations under the Lease, and that in any right of action that shall accrue to Landlord under the Lease, Landlord may, at its option, proceed against Guarantor, without having commenced any action or having obtained any judgment against uniQure, any other guarantor, or any other party liable for uniQure’s obligations under the Lease. Guarantor’s obligations hereunder shall not be affected or impaired by any relief of uniQure from uniQure’s obligations under the Lease by operation of law or otherwise including, without limitation, in connection with proceedings under the bankruptcy laws now or hereafter enacted, or similar laws for the relief of debtors.

c) Guarantor represents and warrants to Landlord that Guarantor, either directly or indirectly, owns 100% of the ownership interests in uniQure, and Guarantor shall derive material financial benefits from the Lease. If Guarantor is a corporation or other entity, Guarantor represents and warrants to Landlord that the individual or individuals executing this Guaranty on behalf of Guarantor is or are

EXHIBIT 4, PAGE 3

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duly authorized to execute and deliver this Guaranty on behalf of Guarantor, that this Guaranty is a valid and binding obligation of Guarantor enforceable in accordance with its terms, and that this Guaranty violates no law, rule, regulation, agreement or contract applicable to or binding on Guarantor.

d) No assignment or transfer of the Lease shall operate to extinguish or diminish the liability of Guarantor under the Guaranty.

e) Guarantor hereby consents to the jurisdiction of the Superior Court of Middlesex County, Massachusetts, in any action, suit or proceeding which Landlord may at any time wish to file in connection with this Guaranty. Guarantor hereby agrees that any action, suit or proceeding to enforce this Guaranty shall be brought in any State or Federal Court in the Commonwealth of Massachusetts and hereby waives any objection which Guarantor may have to said venue; provided, however, that the provisions of this Section shall not be deemed to preclude Landlord from filing any such action, suit or proceeding in any other appropriate forum. Guarantor agrees that service of process may be made, and personal jurisdiction over Guarantor obtained, by service of a copy of the summons, complaint and other pleadings required to commence such litigation upon its agent. Guarantor hereby designates its agent to be: [Corporation Service Company, 84 State Street, Boston, MA 02109]. Guarantor agrees that this appointment of an agent for service of process is made for the mutual benefit of Guarantor and Landlord and may not be revoked without Landlord’s prior written consent. Guarantor hereby consents to the enforcement of any judgment obtained by Landlord with respect to this Guaranty in the Commonwealth of Massachusetts or any other state or country in which Guarantor does business or maintains assets..

f) It shall be deemed a material default by Guarantor hereunder if any proceeding shall be instituted pursuant to any of the provisions of any law in the jurisdiction in which Guarantor is organized relating to bankruptcy, reorganizations, arrangements, compositions or other relief from creditors (a) by Guarantor, or (b) against Guarantor if Guarantor shall fail to have such proceedings dismissed within sixty (60) days or if Guarantor is adjudged bankrupt or insolvent as a result of any such proceeding. Guarantor shall give Landlord written notice regarding any such proceeding promptly after Guarantor first receives notice of the institution of any such proceedings.

g) Capitalized terms not otherwise defined herein shall have the meanings ascribed to them in the Lease.

h) For any matter relating to procedural or substantive law, this Guaranty shall be construed and enforced according to the internal laws of the Commonwealth of Massachusetts without reference to conflict of laws. If any provision of this Guaranty, or any paragraph, sentence, clause, phrase, or word, or the application thereof, is held invalid in any circumstance, the validity of the remainder of this Guaranty shall be construed as if such invalid part were never included herein. The headings of sections and paragraphs in this Guaranty are for convenience only and shall not be construed in any way to limit or define the content, scope, or intent of the provisions hereof. As used in this Guaranty, the singular includes the plural, and masculine, feminine and neuter pronouns are fully interchangeable, where the context so required.

**I) GUARANTOR HEREBY WAIVES THE RIGHT TO TRIAL BY JURY IN ANY ACTION OR PROCEEDING THAT MAY HEREAFTER BE INSTITUTED BY LANDLORD AGAINST GUARANTOR WITH RESPECT TO THIS GUARANTY.**

EXHIBIT 4, PAGE 4

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**IN WITNESS WHEREOF,** Guarantor executes this Guaranty as an instrument under seal as of the day and year first written above.

uniQure BV

By: \_\_\_\_\_  
\_\_\_\_\_  
\_\_\_\_\_

By: \_\_\_\_\_  
\_\_\_\_\_  
\_\_\_\_\_

EXHIBIT 4, PAGE 5

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## FORM OF LETTER OF CREDIT

BENEFICIARY:

ISSUANCE DATE:

&lt; &gt;

[LANDLORD]

IRREVOCABLE STANDBY  
LETTER OF CREDIT NO.

ACCOMTEE/APPLICANT:

MAXIMUM/AGGREGATE  
CREDIT AMOUNT:  
USD: \$ .

&lt; &gt;

[TENANT]

LADIES AND GENTLEMEN:

We hereby establish our irrevocable letter of credit in your favor for account of the applicant up to an aggregate amount not to exceed and /100 US Dollars (\$ . ) available by your draft(s) drawn on ourselves at sight bearing the clause "Drawn under Irrevocable Standby Letter of Credit Number " and indicating the amount to be drawn down and whether payment should be made by wire transfer (including wiring instructions) or by certified check (including mailing address) accompanied by the original of this Letter of Credit and all amendments, if any, and Beneficiary's signed statement reading as follows:

"WE ARE ENTITLED TO DRAW ON THE LETTER OF CREDIT PURSUANT TO THE TERMS OF THAT CERTAIN LEASE DATED BY AND BETWEEN BENEFICIARY, AS LANDLORD, AND APPLICANT, AS TENANT, FOR PREMISES LOCATED AT , MASSACHUSETTS."

or

"BENEFICIARY HAS RECEIVED NOTICE FROM THAT LETTER OF CREDIT NO. WILL NOT BE AUTOMATICALLY EXTENDED AND IT HAS NOT RECEIVED A REPLACEMENT OF THIS LETTER OF CREDIT FROM APPLICANT SATISFACTORY TO THE BENEFICIARY AT LEAST 60 DAYS PRIOR TO THE EXPIRATION DATE OF THIS LETTER OF CREDIT."

The original Letter of Credit and all amendments, if any, shall be returned to you unless fully utilized.

Unless otherwise stated, all correspondence, documents and sight drafts are to be sent via facsimile to ( ) - with originals to follow by hand delivery with receipted delivery, nationally recognized overnight courier with receipted delivery or certified mail, return receipt requested to our counters at <address>. The date of presentment of any draw shall be the date copies of the Letter of Credit and sight draft are faxed by Beneficiary to <bank>.

You shall have the right to make partial draws against this Letter of Credit, from time to time.

You shall be entitled to assign your interest in this Irrevocable Standby Letter of Credit from time

EXHIBIT 5, PAGE 1

to time to your lender(s) and/or your successors in interest without our approval and without charge. In the event of an assignment, we reserve the right to require reasonable evidence of such assignment as a condition to any draw hereunder.

We shall not recognize any transfer of this Letter of Credit until this original Letter of Credit, together with any amendments, and a signed and completed transfer form in the form attached hereto as Attachment A, is received by us. Under no circumstances shall this Letter of Credit be transferred to any person or entity with which U.S. persons or entities are prohibited from conducting business under U.S. Foreign Asset Control Regulations and other applicable U.S. laws and regulations.

Except as otherwise expressly stated herein, this Letter of Credit is subject to the "Uniform Customs and practice for Documentary Credits, International Chamber of Commerce, Publication No. 500 (1993 Revision)".

This Letter of Credit shall expire at our office on , 20 (the "**Stated Expiration Date**"). It is a condition of this Letter of Credit that the Stated Expiration Date shall be deemed automatically extended without amendment for successive one (1) year periods from such Stated Expiration Date, unless at least sixty (60) days prior to such Stated Expiration Date (or any anniversary thereof) we shall send a written notice to you, with a copy to Goulston & Storrs, 400 Atlantic Avenue, Boston, MA 02110, Attention: Colleen P. Hussey and to the Accomtee/Applicant, by hand delivery, nationally recognized overnight courier with receipted delivery or by certified mail (return receipt requested) that we elect not to consider this Letter of Credit extended for any such additional one (1) year period. In the event that this Letter of Credit is not extended for an additional period as provided above, you may draw the entire amount available hereunder.

If at any time prior to presentation of documents for payment hereunder, we receive a notarized certificate signed by one who purports to be a duly authorized representative on your behalf to execute and deliver such certificate, stating that this Letter of Credit has been lost, stolen, damaged or destroyed, we will mail you a "Certified True Copy" of this Letter of Credit, which shall be treated by us as an original.

In order to cancel this Letter of Credit prior to expiration, you must return this original Letter of Credit and any amendments hereto to our counters with a statement signed by you stating that the Letter of Credit is no longer required and is being returned to the issuing bank for cancellation.

We hereby agree with the drawers, endorsers and bonafide holders that the drafts drawn under and in accordance with the terms and condition of this Letter of Credit shall be duly honored upon presentation.

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EXHIBIT 6  
LANDLORD'S SERVICES

- Electricity and lighting for Common Areas
- Management and administrative services
- Landscaping and grounds maintenance
- Cleaning of Common Areas
- HVAC to Common Areas in customary amounts for similar first-class office and laboratory buildings
- Snow and ice removal for walks, drives and parking areas
- Provide connections to fire protection and life safety system
- Electrical service to the Building's 1600 Amp — 480V 3Ph service and 400 Amp -480 V 3Ph service installed as part of Phase I of Landlord's Work and, from such service, to the location of Tenant's electrical closet with distribution panel and disconnect switch for each of the 2 services
- Gas service via a 3" natural gas line to the Premises
- Municipal water service via a 2" domestic water service with backflow preventer to the Premises

All of Landlord's services shall be provided in a manner consistent with similar services in comparable first class office and laboratory buildings in the area.

EXHIBIT 6, PAGE 1

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EXHIBIT 7  
TENANT'S HAZARDOUS MATERIALS

- 1 M NaOH; 96% EtOH (to prepare a 20% solution) for cleaning and storage of Akta Pilot
- 85% phosphoric acid (to prepare a 0.1 M solution) as part of a chromatography buffer solution
- Acetone as part of a chromatography buffer solution. The buffer solutions used in the 50 L scale process are in the range of 2 — 5 L volume
- Small volumes of DMSO and glutar aldehyde for cell bank and fixing samples for EM, respectively
- Ethidium bromide (small volumes)
- Beta-mercapto ethanol (small volumes)
- Methanol for RP-HPLC (larger volumes — liter amounts)

EXHIBIT 7, PAGE 1

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EXHIBIT 8  
RULES AND REGULATIONS

**A. General**

1. Tenant and its employees shall not in any way obstruct the sidewalks, halls, stairways, or exterior vestibules of the Building, and shall use the same only as a means of passage to and from their respective offices. At no time shall Tenants permit its employees, contractors, or other representatives to loiter in Common Areas or elsewhere in and about the Property.
2. Corridor doors, when not in use, shall be kept closed.
3. Areas used in common by tenants shall be subject to such reasonable regulations as are posted therein.
4. Any Tenant or vendor sponsored activity or event in any Common Area must be approved and scheduled through Landlord's representative, which approval shall not be unreasonably withheld.
5. No animals, except Seeing Eye dogs, shall be brought into or kept in, on or about the Premises or Common Areas.
6. Alcoholic beverages (without Landlord's prior written consent), illegal drugs or other illegal controlled substances are not permitted in the Common Areas, nor will any person under the influence of the same be permitted in the Common Areas. Landlord reserves the right to exclude or expel from the Building any persons who, in the judgment of the Landlord, is under the influence of alcohol or drugs, or shall do any act in violation of the rules and regulations of the Building.

7. No firearms or other weapons are permitted in the Common Areas.
8. No fighting or "horseplay" will be tolerated at any time in the Common Areas.
9. Tenant shall not cause any unnecessary janitorial labor or services in the Common Areas by reason of Tenant's carelessness or indifference in the preservation of good order and cleanliness.
10. Smoking and discarding of smoking materials by Tenant and/or any Tenant Party is permitted only in exterior locations designated by Landlord. Tenant will instruct and notify its employees and visitors of such policy.
11. Bicycles and other vehicles are not permitted inside or on the walkways outside the Building, except in those areas specifically designated by Landlord for such purposes
12. Tenant shall not operate or permit to be operated on the Premises any coin or token operated vending machine or similar device (including, without limitation, telephones, lockers, toilets, scales, amusement devices and machines for sale of beverages food, candy, cigarettes or other goods), except for those vending machines or similar devices which are for the sole and exclusive use of tenant's employees.
13. Canvassing, soliciting, and peddling in or about the Building is prohibited. Tenant, its employees, agents and contractors shall cooperate with said policy, and Tenant shall cooperate and use best efforts to prevent the same by Tenant's invitees.
14. Fire protection and prevention practices implemented by the Landlord from time to time in the

Common Areas, including participation in fire drills, must be observed by Tenant at all times.

15. Except as provided for in the Lease, no signs, advertisements or notices shall be painted or affixed on or to any windows, doors or other parts of the Building that are visible from the exterior of the Building unless approved in writing by the Landlord.
16. The restroom fixtures shall be used only for the purpose for which they were constructed and no rubbish, ashes, or other substances of any kind shall be thrown into them. Tenant will bear the expense of any damage resulting from misuse.
17. Tenant shall utilize a pest control service reasonably approved by Landlord to control pests in the Premises. Except as included in Landlord's Services, tenants shall bear the cost and expense of such pest control services.
18. Tenant shall not install, operate or maintain in the Premises or in any other area of the Building, any electrical equipment which does not bear the U/L (Underwriters Laboratories) seal of approval, or which would overload the electrical system or any part thereof beyond its capacity for proper, efficient and safe operation as determined by Landlord, taking into consideration the overall electrical system and the present and future requirements of the Building.
19. Tenants shall not perform improvements or alterations within the Building or their Premises, if the work has the potential of disturbing the fireproofing which has been applied on the surfaces of structural steel members, without the prior written consent of Landlord, subject to the provisions of the Lease.
20. Tenants shall manage its waste removal program, at its sole cost and expense, keeping any recyclables, garbage, trash, rubbish and refuse in vermin-proof containers for Tenants sole use within the Landlord designated area until removed.
21. Lab operators who travel outside lab space must abide by the one glove rule and remove lab coats where predetermined.
22. Chemical lists and MSDS sheets must be readily available at the entrance to each lab area. In the event of an emergency, first responders will require this information in order to properly evaluate the situation.
23. Tenant shall provide Landlord, in writing, the names and contact information of two (2) representatives authorized by Tenant to request Landlord services, either billable or non-billable and to act as a liaison for matters related to the Premises.

## **B. Access & Security**

1. Tenant shall not place any additional lock or locks on any exterior door in the Premises or Building or on any door in the Building core within the Premises, including doors providing access to the telephone and electric closets and the slop sink, without Landlord's prior written consent. A reasonable number of keys to the locks on the doors in the Premises shall be furnished by Landlord to Tenant at the cost of Tenant, and Tenant shall not have any duplicate keys made. All keys shall be returned to Landlord at the expiration or earlier termination of this Lease.
2. Landlord may from time to time adopt appropriate systems and procedures for the security or safety of the Building, its occupants, entry and use, or its contents, provided that Tenant shall have access to the Building 24 hours per day, 7 days a week. Tenant, Tenant's agents, employees, contractors, guests and invitees shall comply with Landlord's reasonable

requirements relative thereto. Tenant acknowledges that Property security problems may occur which may require the employment of extreme security measures in the day-to-day operation of the Common Areas. Accordingly, Tenant agrees to cooperate and cause its employees, contractors, and other representatives to cooperate fully with Landlord in the implementation of any reasonable security procedures concerning the Common Areas.



3. Tenant and its employees, agents, contractors, invitees and licensees are limited to the Premises and the Common Areas. Tenants and its employees, agents, contractors, invitees and licensees may not enter other areas of the Project (other than the Common Areas) except when accompanied by an escort from the Landlord.

#### C. Shipping/Receiving

1. Dock areas for the Building shall not be used for storage or staging by Tenant.
2. In no case shall any truck or trailer be permitted to remain in a loading dock area for more than forty-eight (48) hours.
3. There shall not be used in any Common Area, either by Tenant or by delivery personnel or others, in the delivery or receipt of merchandise, any hand trucks, except those equipped with rubber tires and sole guards.
4. Lab operators carrying any lab related materials may only travel within the Premises. At no time should any lab materials travel in the Common Areas.
5. Any dry ice brought into the building must be delivered through the loading dock serving the Premises.
6. All nitrogen tanks must travel through the loading dock serving the Premises and should never be left unattended outside of the Premises.

### CONTRACTOR

#### RULES AND REGULATIONS

##### Construction/Remodeling

Lincoln Property Company's construction work procedure is designed to provide efficient scheduling of work while protecting other tenants from unnecessary noise and inconvenience.

The Lincoln Property Company Construction Rules and Regulations; Hot Work Protocol and Hot Work Checklist attached to this document contain detailed information to assist you and your General Contractor in planning construction projects. Please review it carefully before design begins.

Please note that the summary below highlights key aspects of the **Detailed Construction Rules and Regulations** for your convenience and does not supersede it in any way.

##### Summary:

Before starting a project — contact the Lincoln Property Company Management Office at (781) 890-9800. The Property Manager will be happy to assist you in completing your project efficiently.

Use reasonable efforts to incorporate the provisions of the attached document into all of your agreements and contracts. You will need written approval from the Property Manager before contracting any work to the extent provided in the Lease.

Before construction provide two (2) sets of drawings and plans to the Property Manager for approval. The Property Manager must also approve your list of contractors and subcontractors to the extent provided in the Lease.

Schedule a pre-construction meeting with the Property Management Team. Meeting materials should include detailed schedules; addresses and telephone numbers of supervisors, contractors and subcontractors; copies of permits; proof of current insurance (including all subcontractor); payment, performance and lien bonds where applicable; and notice of any contractor's involvement in a labor dispute.

We expect all contractors to maintain safe and orderly conditions, labor harmony, and proper handling of any hazardous materials. MSDS sheets must be supplied to the management office. We may stop any work that does not meet the conditions outlined in the attached document.

Before occupying the completed space, submit the final certificate of occupancy (to the extent available in light of other work in the building) and any other approvals to the Property Manager. We also require an air balancing report signed by a professional engineer and "as built" set of drawings in AutoCAD format on disk and one (1) hard copy showing all of the work in full detail where such work is susceptible to as built plans.

##### Detailed Construction Rules and Regulations:

The following Rules and Regulations have been established by Lincoln Property Company (LPC) and shall be adhered to by all contractors/vendors working the Premises. Building management shall reserve

the right to have any individual(s) or company removed from the building for any violation of these provisions.

<b>Building Management:</b>	Lincoln Property Company (LPC)
<b>Property Manager:</b>	Laura Scialdoni, 781-890-9800 (office)
<b>Regular Business Hours:</b>	Monday through Friday - 8:00 a.m. to 6:00 p.m.

**A. General:**

1. This document will be an Exhibit for all construction contracts for work at this property.
2. No construction or alterations of the property may be started without the prior approval of LPC subject to the terms of the Lease. Prior to the requested start of work, the contractor must submit to LPC a full set of stamped architectural drawings for approval, reflecting the full and complete scope of the project.
3. LPC requires copies of certificates of insurance for all contractors working inside the building per the buildings insurance requirements.
4. LPC requires that the general contractor provide a project superintendent licensed by the Department of Public Safety and hold a current license designation of "Licensed Construction Supervisor". The project superintendent shall be on site every day when construction activities are in progress and on site for all after hours work.
5. Prior to the commencement and upon completion of each project, LPC and the contractor will walk-through the public areas, i.e., restrooms, common corridors, stairwells, etc. Any prior damage will be noted. Any subsequent damage to the surrounding areas will be the responsibility of the general contractor to repair.
6. The contractor is responsible for filing and obtaining all required local building, fire and/or utility permits, as applicable, prior to the commencement of any work and must be licensed or certified to perform all work where specified or required by law. The contractor shall comply with all inspectional services and fire department requirements related to the issuance of the building permits and shall display the building permit and inspection records as required by building code. Where applicable, permits are to be posted as directed by LPC.
7. The contractor shall not borrow any materials, i.e., tools, extension cords, dollies, ladders, etc., from the maintenance department. The contractor is not allowed access to the maintenance shop or storage closets. Prior authorization, which shall not be unreasonably withheld, is required from LPC for access to any electric, plumbing, telephone or HVAC closets. Should access be permitted, the contractor will be responsible for any damage.
8. All work undertaken by contractors on site must be performed in accordance with safety standards, which include, but are not limited to, compliance with Occupational Safety and Health Administration (OSHA). Contractor's safety procedures may exceed OSHA standards but in no case shall they fail to meet these minimum requirements.

EXHIBIT 8, PAGE 2

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9. All accidents, disturbances, labor disputes or threats thereof, and other noteworthy events pertaining to the building or a tenant's property shall be reported to LPC. A written report must follow within 24 hours.

**B. Building Permits & Certificates of Occupancy**

1. A copy of the building permit, if one is required by law, must be delivered to LPC prior the start of any construction project and the permit card must be posted on the construction site in full view at all times.
2. A copy of the fully executed building permit, showing all final inspection sign-offs must be delivered to LPC prior to the receipt of the certificate of occupancy, if one is required by law.
3. Any original certificate of occupancy must be delivered to LPC as soon as it is issued.

**C. Building Standards**

1. All materials and ME/P equipment shall conform to the approved plans.
2. All window blind systems shall be reasonably approved by Landlord to be consistent with existing blind systems\.

**D. Construction Work Rules**

1. The general contractor will be responsible for providing fire extinguishers throughout the construction area. There should be one fire extinguisher every seventy-five feet within the construction area. Base building fire extinguishers may not be removed from stairwell cabinets for construction purposes.
2. The contractor must remove an exterior window and install a painted plywood panel that will allow dust control by a contractor-provided HEPA filter unit. The window will be installed in an area that will not affect the construction or new interior walls. The general contractor will provide an electrical outlet for the HEPA filter unit. The general contractor will be responsible to clean any spaces in the building that become contaminated with construction dust. The HEPA filter will be operational 24/7 during the construction project.
3. Work shall be scheduled in accordance with the Lease. No new construction work shall take place or other trades be permitted access to the construction area until the construction area is completely demolished. All debris is to be removed daily.
4. All construction involving high levels of noise, including, but not limited to, coring, drilling, ram setting, shooting of floor track or ceiling track must be performed between the hours of 6:00 p.m. and 7:30 a.m. Monday through Friday or anytime on a scheduled weekend.
5. All spray painting and staining is to be performed after normal business hours and is to be coordinated with LPC to ensure proper ventilation if determined that this work will impact the other tenants in the building. Large spray areas (3,000 sf plus) must be done on a Saturday to provide at least two days of ventilation. All oil base painting must be performed over a weekend period.

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6. All work requiring the shutdown associated with electrical, mechanical, sprinkler or plumbing work outside the Premises shall be supervised by a representative of LPC, the cost of which shall be charged directly to Tenant at the prevailing building rate.
7. If work is required to be performed in adjacent tenant space, this work shall be performed after normal business hours and shall be supervised by a building engineer. The LPC engineer's cost will be a Tenant expense.
8. The contractor shall provide a workbox and pull strings for telephone work. Tel/data work is by the tenant's contractor.
9. All contractors working in the building are required to provide their own ladders with a rubber shoe on each leg. Ladders shall be certified to meet OSHA and ANSI standards. All push carts and dumpsters shall have rubber tires to reduce the construction noise.
10. The general contractor's vacuums shall have filters in place and be in good working condition.
11. The contractor shall install temporary partitions (sheet rock) for security purposes and site protection in any public corridor where doors are being relocated or moved. Temporary access doors for construction areas connecting with a public corridor will be building standard, i.e., door, frame, hardware and lockset, with a copy of the key to be furnished to LPC.
12. No building entry, suite entry, stairwell or any other applicable common area of building closet doors are to be propped open under any circumstance.
13. The general contractor shall inform LPC in advance if there will be any odor producing construction work, i.e., IDEA wall paint or floor adhesives. The contractor will inform LPC if high VOC products are being utilized. 113 Hartwell Avenue promotes green practices and all materials shall be low VOC. LPC reserves the right to stop this work if the work creates a disturbance to other building tenants.

#### **E. Safety and Protection of Property**

1. Contractors shall police ongoing construction operations and activities at all times, keeping the premises orderly, maintaining cleanliness in and about the premises, and ensuring safety and protection of all areas, including loading docks, elevators, lobbies and all other public areas which are used for access to the premises.
2. Construction materials shall only be stored in the premises where they are to be installed. No storage of materials will be permitted in any public areas, loading docks or corridors leading to the premises, nor in any mechanical rooms, electrical rooms, etc. Materials left in unauthorized areas may be disposed of by LPC.
3. LPC assumes no responsibility for tools, materials or equipment stored at the building.
4. Contractors shall provide adequate protection to all carpets, wall surfaces, doors and trim in all public areas through which materials are transported. Contractors shall continuously clean such areas. Protective measures shall include runners over carpet, padding in elevators and any other

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measures determined by LPC. Any damage to existing walls, carpets, doors or trim during construction shall be repaired by the contractor to the satisfaction of LPC.

#### **F. Parking**

Tenant shall not permit any parking in the loading docks serving other tenants of the Building. Tenant acknowledges that Landlord may enforce the carpool and handicapped parking designations, including the right to have violating vehicles towed at the expense of the owner of such vehicles.

#### **G. Conduct**

1. While in or about the building, all contractors shall perform in a dignified, quiet, courteous and professional manner at all times. Contractors shall wear clothing suitable for their work and shall remain fully attired at all times.
2. No smoking is allowed in the building. Smoking is permitted only in designated outdoor areas at a minimum of 25 feet away from any public entrances. At no time is smoking permitted in the loading dock, stairwells, basement, within the construction area or any other area within the building.
3. The use of alcohol, narcotics and/or controlled substances is strictly prohibited on site, as well as firearms, ammunition, cameras, and any recording devices. Any contractor or their employee found in violation of these regulations will be asked to leave the building.
4. Radios are not permitted on the work site.

#### **H. ME/P Work Rules**

1. All ME/P work shall comply with requirements of local building code, building department, building management rules and regulations and all authorities having jurisdiction. The contractor is to inform the engineer of record and LPC of any existing work or materials which violate any of the above laws and regulations. Any work done by the contractor causing such violation shall be corrected at the contractor's expense.
2. All work performed shall be in accordance with the latest version of NEPA, NEC, NESC and with all applicable state and local codes.
3. The contractor shall perform all city inspections as required and obtain all equipment use permits as required by state and local authorities. Permits shall be turned over to LPC at job completion.

#### **I. Fire Protection Work**

1. The contractor shall provide LPC with a copy of the sprinkler permit prior to the commencement of any work.

2. Sprinkler heads can be relocated during normal business hours if approved by the Lexington Fire Department and Building Inspector. The contractor shall schedule the drain down with LPC at least 24 hours in advance and will provide LPC with the time for the system fill, so that it can be scheduled with the building's fire alarm contractor.

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3. The contractor must be on site before any impairment to the system is made.
4. The sprinkler systems must be restored to full service by the end of the business day. Sprinklers shall not be left impaired overnight.
5. The contractor shall inspect the system for any leaks and remain on site until the system has full operating pressure and the fire alarm panel is free from any trouble conditions.

**J. Fire Alarm Work**

1. The building's fire alarm contractor is BEF Alarms. The general contractor's electrical contractor can install the fire alarm devices and branch wiring, but all final wiring, programming of the FA panel and Town of Lexington testing shall be performed and coordinated by the building's fire alarm contractor. The building's fire alarm contractor should be a subcontractor to the project's electrical contractor. All fire alarm system testing is to be performed after normal business hours.

**K. Loading Dock**

1. All material deliveries must be made through the loading dock and must be transported directly to the job site. The contractor may not use the passenger elevators for the transportation of materials at any time.
2. All vehicles are to be removed from the dock as soon as the delivery is complete. Unattended vehicles will be towed at the contractor's expense.

**L. Salvage and Waste Removal**

1. The contractor is responsible for disposing of all construction debris. The building's trash/recycling compactor is not available. The contractor must make arrangements with LPC for the scheduling and location of all dumpsters. The contractor is to place protection board under the dumpster and is responsible for any damage to the loading dock's deck. The loading dock floor is to be swept and washed by the contractor at the completion of each shift.

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*\*LPC Hot Work Protocol for construction renovation or building repairs dated October 26, 2010 below. This HW protocol is to be followed by all contractors working on the Premises.*

**LINCOLN PROPERTY COMPANY**

**HOT WORK PROTOCOL**

**CONSTRUCTION RENOVATIONS AND BUILDING REPAIRS**

**Definition:**

Hot work is any work activity that generates a flame, heat or sparks such as soldering, brazing, welding, or torch cutting and grinding.

Before any hot work shall commence the following Fire Safety Precautions must be taken:

**1. Notification**

Notify building management, in writing, 72 hours in advance of the location and purpose of planned hot work in the facility. Any heavy hot work, such as structural work or confined space welding, shall take place after normal business hours. Refer to the building's rules and regulations for the defined normal business hours.

**2. Permit**

Provide building management with a copy of the hot work permit from the local authority.

**3. Sprinkler and Fire Alarm System Impairment**

The sprinkler system in the area of the hot work shall not be impaired during the hot work time period. The fire alarm system may only be impaired or zoned out in the area of the hot work with express permission of the local authority.

**4. Fire Watch**

A designated person from the local fire department authority shall be present during all hot work. The person performing the fire watch shall be completely informed of the work to be undertaken and be in radio or telecommunications contact with the local fire station.

**5. Hot Work Area Condition & Preparation**

Floors are to be swept clean. Any accumulation of dust shall be removed. Combustible materials shall be removed from the area of hot work and the hot work area shall be properly ventilated. Hot work is not to be conducted in the presence of flammable gases, vapors, liquids or dusts. If hot work is to be performed in a confined area atmospheric testing shall be performed prior to and during the hot work procedure to ensure the work site atmosphere is below the low explosive limit.

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6. Person(s) Conducting Hot Work

The person conducting hot work shall be certified, fully trained and competent in use of the equipment and wear the appropriate personal protective equipment.

7. Fire Fighting Equipment & Site Preparation

Fire extinguishers shall be present and within reach during all hot work. The person conducting hot work and fire watch personnel shall be informed of the location and have access to all fire-fighting equipment. Shields are to be erected where electric welding is to take place to prevent ultra-violet light exposure to others in the area. Floor or wall openings located 10 feet or less from the work site are to be covered to prevent hot sparks from entering walls or shafts and falling to floors below.

8. Fire Watch Standby

Fire watch personnel shall remain on site 20 minutes after hot work has been completed. Notification shall be sent to the local authorities after hot work is completed. Fire alarm system point zoning shall be restored to active condition after work area is ventilated.

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**LINCOLN PROPERTY COMPANY**

**Hot Work Protocol  
Check List**

1. Contractor Compliance  
Is contractor in compliance with LPC protocol?
2. Type of hot work  
Welding, cutting, grinding-soldering, structural
3. Location of hot work
4. Date and time to be performed
5. Permit provided to LPC
6. Certificate of Insurance for all contractors and subcontractors presented to building management
7. Is a fire watch required by local authority?
8. Any fire protection sprinklers impaired?
9. Fire alarm system to be temporarily zoned out in work area
10. Work area free of combustible equipment?
11. Work area properly ventilated prior to and during hot work
12. All floor and wall openings covered and protected to prevent sparks and slag from traveling to other unprotected shafts and voids to the floor below
13. Portable fire extinguisher onsite and fully charged
14. Person(s) onsite performing hot work are qualified and have proper protective clothing and face shields
15. Person conducting fire watch from local authority is on site and is in telecommunication contact with fire chief on duty

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EXHIBIT 9

[Insert property address], Massachusetts

FORM OF NOTICE OF LEASE

NOTICE OF LEASE

Notice is hereby given pursuant to Chapter 183, Section 4 of the General Laws, of a lease upon the following terms:

Landlord:

Tenant:

Date of Lease Execution: , , 200

Premises: . The land on which the Premises are located is more particularly described on Exhibit A attached hereto and incorporated herein.

Term and Commencement Date: Approximately ( ) years, commencing on , 20 and expiring on , 20 .

Extension Options: ( ) extension options of ( ) years each.

Expansion Options: Subject to the terms and conditions of the Lease, Tenant has (a) a right of second offer to lease certain portions of the Building, which right of second offer expires no later than April 1, 2014, and (b) a right of first offer to lease any portion of the Building outside the Premises.

*This Notice of Lease has been executed merely to give notice of the Lease, and all of the terms, conditions and covenants thereof which are incorporated herein by reference. The parties hereto do not intend this Notice of Lease to modify or amend the terms, conditions and covenants of the Lease.*

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[Insert property address], Massachusetts

EXHIBIT 9, PAGE 2

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Executed as an instrument under seal this day of , 20 .

LANDLORD:	TENANT:
By: _____	By: _____
Name: _____	Name: _____
Title: _____	Title: _____

[ADD NOTARY BLOCKS]

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EXHIBIT 10

PTDM

Parking and Transportation Demand Management Plan (PTDM)

113 Hartwell Avenue, Lexington, MA

EXHIBIT 10, PAGE 1

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Table of Contents

- I. Project Description
- II. Proposed PTDM Plan
- III. Alternative Mode Promotions/Incentives
- IV. Parking Management/SOV Disincentives
- V. Marketing Programs

I. Project Description

King 113 Hartwell LLC Street acquired 113 Hartwell Avenue, a one-story R&D building located in Lexington, Massachusetts, formerly occupied by Instrumentation Laboratories. The existing building is 103,630 square feet and sits on 10 acres. King 113 Hartwell LLC intends to reposition this building for life science/laboratory use. Base building renovations include new HVAC and electrical service to accommodate laboratory use. The renovation

will also include an architectural treatment to the façade of the building. The landscape and plantings on the site will also be redesigned in order to allow for more usable outdoor space.

### **Parking and Transportation**

The likely place of origin for employees commuting to 113 Hartwell Avenue is Lexington, Cambridge, Boston, surrounding communities (i.e., Arlington, Belmont, Bedford, Brookline, Watertown), North Shore, Metro West, South Shore and Southern New Hampshire.

There is currently parking in surface lots on-site for XXX cars. The redevelopment of the building allows parking for XXX cars, but King 113 Hartwell LLC will only stripe a total of XXX parking spots, a net decrease of XX parking spots. King 113 Hartwell LLC will discourage the use of cars as a primary mode of transportation but if tenant demand requires, the owner reserves its right to stripe the XXX parking spots along the back of the parking lot as depicted in the attached plan.(XXXXXX)

The Hartwell Avenue area is currently serviced by the MBTA Bus Routes 62 (Bedford V.A Hospital-Alewif Station) and 76 (Hanscom/Lincoln Lab-Alewif Station).

## **II. Proposed Parking and Transportation Demand Management Plan**

King 113 Hartwell LLC will develop and implement a comprehensive mitigation program designed to minimize the amount of single occupant vehicles (SOV) entering the site. The components of the mitigation program included herein address the Parking and Transportation Demand Management Plan (PTDM) and other incentives which should encourage tenants to use an alternate mode of transportation.

King 113 Hartwell LLC will take an active role in pursuing measures to help mitigate and reduce traffic volumes on Hartwell Avenue. Primary transportation options that will be encouraged include ridesharing and bicycle/pedestrian trips.

We will work closely with our tenants to encourage their ongoing active participation in our efforts to mitigate traffic, and will offer incentives and prizes to employees who choose to participate in these programs.

### **Bicycle/Pedestrian Trips**

The building renovation plans for 113 Hartwell Avenue include the installation of new bike racks which will accommodate the required number of bikes.

In order to encourage employees to use bicycles and or walk, 113 Hartwell Avenue will have shower and changing facilities on-site, and will provide tenants with shampoo/conditioner and shower gel at no cost.

The building will also provide shared umbrellas and parkas for use in inclement weather.

King 113 Hartwell Avenue LLC will purchase 10 building bicycles, which tenants will be able to use, free of charge, during business hours. We anticipate these bicycles will be used to pick-up lunch, or do local errands such as going to the bank or post office.

### **Benefits of Bicycle/Pedestrian Trips**

- Less automobile traffic congestion
- Reduced fuel consumption
- Better air quality
- Less expensive than driving
- Fitness benefits to the cyclist

### **Rideshare Program**

King 113 Hartwell LLC will establish a Rideshare Program for the building. Our Transportation Coordinator will be responsible for creating an electronic board, where tenants can post information and communicate with other commuters to arrange for transportation. Initially this program will focus on ride matching with other employees in the building, but if necessary could expand to include other tenants in the area. King 113 Hartwell will work with our tenants to create a Place of Residence Database, which will initially help employees connect with other commuters in their immediate area.

King 113 Hartwell will designate 5% of the total parking spots for rideshare vehicles to help encourage participation in the program.

### **Benefits of Rideshare Program**

- Less automobile traffic congestion
- Reduced fuel consumption
- Better air quality
- Less expensive than SOV because of shared transportation costs
- Less travel time if carpool/HOV lanes can be utilized
- More relaxing because of shared driving responsibilities

### **Ongoing Management**

Critical to ensuring the success of our PTDM Plan is the ongoing management of the program. Management activities will include, but not be limited to the following:

- Appoint a Transportation Coordinator whose responsibilities will include the following:
  - Coordinate Rideshare Board/Postings
  - Create a 113 Hartwell Avenue Facebook Page (or similar on-line web site), which tenants can use to communicate regarding rideshares

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- Provide tenants with updated information and options on alternative modes of transportation
- Promote transportation options through events such as bicycle tune-up day, car wash for carpoolers, gas bucks for groups
- Help in the creation of a place of residence database which will connect employees in the building who live in similar locations
- Monitor and evaluate results of the PTDM Plan through tenant surveys
- Ensure that specific language is included in all tenant leases which requires the tenant's participation in the Hartwell Avenue Transportation Management Association
- Take a leadership role in working with other landlords in the area to encourage cooperation and promotion of transportation options
- Become active members in area transportation groups

### **III. Alternative Mode Promotions/Incentives**

As outlined in the PTDM Plan, the primary alternative transportation modes to be encouraged will be bicycle/pedestrian trips and ridesharing. King 113 Hartwell LLC plans to offer the following incentives in order to make these alternative transportation modes more attractive to tenants:

#### **Bicycle/Pedestrian Trips**

- Create bike-friendly areas that take into consideration adequate site-lines at entrances
- Provide bike racks with capacity for the required number of bikes
- Provide shower and changing facilities on-site
- Create financial incentive programs and prizes, most likely based on mileage per year
- Offer free bike tune-up day each spring
- Offer loaner bicycles for errands during the day
- Offer a free pair of sneakers for the employee that walks to work the most number of days each year

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#### **Rideshare Program**

- Designate Preferential Parking for ridesharing vehicles near tenant entries
- Organize an internal ride match program for employees
- Provide access to information on other ridesharing programs in the area
- Provide incentives to employees who commute by ridesharing, such as "Gas Bucks for Groups". Once a quarter employees who participate in ridesharing are eligible for free "Gas Bucks", a gift card towards their next gas purchase.
- Provide free car washes once a year at the "Car Wash for Carpoolers" event
- Provide other prize drawings

### **IV. Parking Management/SOV Disincentives**

The primary way in which SOV use will be discouraged at 113 Hartwell Ave is through the reduction of parking spaces. The Parking Ratio for tenants will only be 3.3/1000. The number of parking spots for SOV vehicles will initially be reduced by XX and further reduced by the Preferential Parking which will be given to rideshare vehicles. 5% of the total parking spots will be dedicated for rideshare vehicles. Additionally tenants who choose to commute in SOV;s will not be eligible for any incentive programs such as the "Gas Bucks for Groups" or "Car Wash for Carpoolers".

### **V. Marketing Programs**



King 113 Hartwell LLC will actively promote the use of alternate modes of transportation through a comprehensive marketing plan. The marketing plan will focus on the following messages, which we are confident will help change travel modes:

- *Cost Savings*-Ridesharing reduces transportation costs
- *Health Benefits*-Riding your bike to work or walking is a great way to get exercise into your daily routine
- *Enhanced Convenience*-Shower facilities and bike-racks on-site make it easy to trade in 4 wheels for 2
- *Environmentally Friendly*-Better air quality, reduced fuel consumption

King 113 Hartwell plans to use a variety of means to convey these messages and promote alternate transportation to its tenants. Included in each tenant orientation packet will be information on the buildings transportation demand management programs, including the Rideshare program. Furthermore, throughout the year, email blasts will be sent to all tenants alerting them to incentives/events (such as free bike tune-up day) encouraging participation in these programs.

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EXHIBIT 11

SIGNAGE GUIDELINES

[SEE 2 PAGES ATTACHED]

EXHIBIT 11, PAGE 1

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EXHIBIT 12

MATTERS OF RECORD

1. Real estate taxes not yet due and payable
2. Matters shown on a survey entitled "King Street Properties, 113 Hartwell Avenue, Lexington, Massachusetts, ALTA/ACSM Land Title Survey Plan," Scale 1" = 50', dated 10/7/10, prepared by Kelly Engineering Group, Inc.
3. Construction Mortgage and Security Agreement from Landlord to Eastern Bank in the original principal amount of \$18,200,000 dated as of January 27, 2011 and filed with the Middlesex South Registry District of the Land Court (the "**Registry**") as Document No. 1557629.
4. Collateral Assignment of Leases and Rents between Landlord and Eastern Bank, its successors and/or assigns, as their interests may appear, dated as of January 27, 2011 and filed as Document No. 1557630.
5. Easements set forth in Taking by the Northeastern Gas Transmission Company dated July 13, 1951 and recorded in Book 7772, Page 162.
6. Rights and Easements set forth in Taking by the United States of America pursuant to Judgment in the United States Court for the District of Massachusetts (Civil Action 4-109-S) dated February 12, 1954, recorded in Book 8219, page 421.
7. Rights and easements associated with the ditches as approximately shown on Plan filed in Registration Book 685, Page 171, at date of original decree (May 17, 1963).
8. Notice of Variance/Special Permit dated September 14, 1967 issued by the Board of Appeals of the Town of Lexington to Thomas A. Rosse, Petitioner on behalf of the Trustees of Hartwell Avenue Realty Trust, Notice of which is dated October 26, 1967 and filed as Document No. 449467.
9. Notice of Variance/Special Permit dated July 1, 1969 issued by the Board of Appeals of the Town of Lexington on Petition of Instrumentation Laboratory, Inc., Notice of which is dated July 24, 1969 and filed as Document No. 467983.
- 10.

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EXHIBIT 13

FORM OF SNDA

**SUBORDINATION, NON-DISTURBANCE AND  
ATTORNMEN T AGREEMENT**

This Subordination, Non-Disturbance and Attornment Agreement (hereinafter, the "Agreement") is made this                      day of                      , 2013, by and among KING 113 HARTWELL LLC (hereinafter, the "Landlord" or "Borrower"), with an address of c/o King Street Properties, 255 Bear Hill Road, Waltham, MA 02451, UNIQUE, INC. (hereinafter, the "Tenant"), with an address of 8 Amanda Lane, Weston, MA 02493, , and EASTERN BANK (hereinafter, the "Mortgagee"), with a principal place of business at 265 Franklin Street, Boston, Massachusetts 02110.

Introductory Provisions

A. Mortgagee is relying on this Agreement as an inducement to Mortgagee in making and maintaining a loan (hereinafter, the "Loan") secured by, among other things, a certain Construction Mortgage and Security Agreement dated as of January 27, 2011 and filed with the Middlesex South Registry District of

the Land Court as Document No. 1557629 (hereinafter, the "Mortgage") given by Borrower covering property commonly known as and numbered 113 Hartwell Avenue, Lexington, Middlesex County, Massachusetts (hereinafter, the "Property").

- B. Tenant is the holder of and tenant under that certain lease (hereinafter, the "Lease") dated July , 2013, made with Landlord, covering certain premises (hereinafter, the "Demised Premises") at the Property.
- C. Mortgagee requires, as a condition to the making and maintaining of the Loan, that the Mortgage be and remain superior to the Lease.
- D. Tenant requires, as a condition to the Lease being subordinate to the Mortgage, that its rights under the Lease be recognized.
- E. Mortgagee, Landlord, and Tenant desire to confirm their understanding with respect to the Mortgage and the Lease.

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NOW, THEREFORE, in consideration of the foregoing, the mutual covenants and agreements contained herein, and other valuable consideration, the receipt and adequacy of which are hereby acknowledged, and with the understanding by Tenant that Mortgagee will rely hereon in making and maintaining the Loan, Mortgagee, Landlord, and Tenant agree as follows:

1. The Lease is subordinate and inferior to the lien of the Mortgage and any renewal, substitution, extension or replacement thereof and each advance made thereunder as though said Mortgage, and each such renewal, substitution, extension or replacement were executed, recorded and the advance made before the execution of the Lease.
2. So long as Tenant is not in default (beyond any period expressed in the Lease within which Tenant may cure such default) in the payment of rent or in the performance or observance of any of the terms, covenants or conditions of the Lease on Tenant's part to be performed or observed, (i) Tenant's occupancy of the Demised Premises shall not be disturbed by Mortgagee, nor will Tenant's rights under the Lease be impaired (except as expressly provided in this Agreement), in the exercise of any of its rights under the Mortgage during the term of the Lease or any extension or renewal thereof, made in accordance with the terms of the Lease, and (ii) Mortgagee will not join Tenant as a party defendant in any action or proceeding for the purpose of terminating Tenant's interest and estate under the Lease because of any default under the Mortgage.
3. In the event any proceedings are brought for the foreclosure of the Mortgage, or if the Property or the Demised Premises are sold pursuant to the power of sale under the Mortgage, Tenant shall attorn to the purchaser upon any such foreclosure sale and shall recognize such purchaser thereafter as the Landlord under the Lease. Such attornment shall be effective and self-operative without the execution of any further instrument on the part of any of the parties hereto. Tenant agrees, however, to execute and deliver at any time and from time to time, upon the request of any holder(s) of any of the indebtedness or other obligations secured by the Mortgage, or upon request of any such purchaser, (a) any reasonable instrument or certificate which, in the reasonable judgment of such holder(s), or such purchaser, may be necessary or appropriate in any such foreclosure proceeding or otherwise to evidence such attornment on the terms and conditions set forth in this Agreement, and (b) an estoppel certificate regarding the status of the Lease, consisting of statements, if true (and if not true, specifying in what respect), (i) that the Lease is in full force and effect, (ii) the date through which rentals have been paid, (iii) the duration and date of the commencement of the term of the Lease, (iv) the nature of any amendments or modifications to the Lease, (v) that to the best of Tenant's knowledge, no default, or state of facts, which with the passage of time, or notice, or both, would constitute a default, exists on the part of either party to the Lease, and (vi) the dates on which payments of additional rent, if any, are due under the Lease.
4. If Mortgagee shall succeed to the interest of Landlord under the Lease in any manner, or if any purchaser acquires the Property, or the Demised Premises, upon any foreclosure of the Mortgage, Mortgagee or such purchaser, as the case may be, shall have the same remedies by entry, action or otherwise in the event of any default by Tenant (beyond any period expressed in the Lease within which Tenant may cure such default) in the payment of rent or in the performance or observance of any of the terms, covenants and conditions of the Lease on Tenant's part to be performed or observed that the Landlord had or would have had if Mortgagee or such purchaser had not succeeded to the interest of the present Landlord. From and after any such attornment, Mortgagee or such purchaser shall be bound to Tenant under all the terms, covenants and conditions of the Lease, and Tenant shall, from and after such attornment to Mortgagee, or such purchaser, have the same remedies against Mortgagee, or such purchaser, for the breach of an agreement contained in the Lease that Tenant might have had under the Lease against Landlord if Mortgagee or such purchaser had not succeeded to the interest of Landlord; provided, however, that Mortgagee or such purchaser shall only be bound during the period of its

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ownership, all Tenant claims shall be satisfied only out of the interest, if any, of Mortgagee or such purchaser in the Property, and Mortgagee and such purchaser shall not be (a) liable for any act or omission of any prior landlord (including the Landlord) provided, that, nothing in this clause (a) being deemed to relieve Mortgagee or any such purchaser of its continuing obligations as landlord under the Lease with respect to the period from and after the date of such succession; or (b) liable for or incur any obligation with respect to the construction of the Property or any improvements therein (provided that nothing in this Agreement shall prohibit or delay Tenant from the exercise of its express remedies set forth in Section 3.4 of the Lease with respect to the Landlord's Contribution (including to the extent affected by Section 3.5) and Section 3.2 of the Lease with respect to Landlord's Work, regardless of when such remedies are triggered or whether the events giving rise to such remedies arose out of the act of Landlord prior to such succession); or (c) subject to any offsets or defenses which Tenant might have against any prior landlord (including the Landlord) (other than offsets expressly provided for in the Lease); or (d) bound by any rent or additional rent which Tenant might have paid for more than one month in advance of the date due to any prior landlord (including the Landlord); or (e) bound by any amendment or modification of any term of the Lease (other than amendments exercising the rights of Tenant that are expressly contemplated by the Lease, such as for extension or expansion) made without Mortgagee's prior written consent, such consent not be unreasonably withheld, conditioned or delayed; or (f) bound by or responsible for any security deposit or prepaid rent not actually received by Mortgagee (Mortgagee acknowledging that, upon actual receipt thereof, it shall hold any cash Security Deposit under the Lease on the terms and conditions set forth in the Lease); or (g) liable for or incur any obligation with respect to any breach of warranties of any nature under the Lease or otherwise including without limitation any warranties respecting use, compliance with zoning, landlord's title, landlord's authority, habitability and/or fitness for any purpose, or possession; or (h) liable for consequential damages.

5. Nothing herein contained is intended, nor shall it be construed, to abridge or adversely affect any right or remedy of the Landlord under the Lease, or any subsequent Landlord, in the event of any default by Tenant (beyond any period expressed in the Lease within which Tenant may cure such default) in the payment of rent or in the performance or observance of any of the terms, covenants or conditions of the Lease on Tenant's part to be performed or observed.

6. Tenant agrees to provide Mortgagee with a copy of each notice of default given to Landlord under the Lease, at the same time as such notice of default is given to the Landlord, and that in the event of any default by the Landlord under the Lease, Tenant will take no action to terminate the Lease unless the default remains uncured for a period of thirty (30) days after written notice thereof shall have been mailed, postage prepaid, to Landlord at Landlord’s address, and to Mortgagee at its address stated in (or pursuant to) Section 7 below; provided, however, that if any such default is such that it reasonably cannot be cured within said thirty-day period, such period shall be extended for such additional period of time as shall be reasonably necessary for Mortgagee to obtain possession of the Property and to foreclose the Mortgage (but in any event not to exceed 180 days in the aggregate), if Mortgagee gives Tenant written notice of Mortgagee’s election to undertake the cure of the default within such 30 day period and if curative action (including, without limitation, action to obtain possession and foreclose) is instituted within such 30 day period and is thereafter diligently pursued. Mortgagee shall have no obligation to cure any default under the Lease prior to the time that it succeeds to the interest of Landlord under the Lease. Nothing in this Section 6 shall be deemed to prohibit or delay Tenant from exercising its express termination rights under the Lease pursuant to Sections 9.5 [interruption], Article 15 [casualty/condemnation], and 3.2 [Landlord’s Work] thereunder on the terms and conditions contained therein.

7. Any notice or communication required or permitted hereunder shall be in writing, and shall be given or delivered by United States mail, registered or certified, postage fully prepaid, return receipt requested, or by recognized courier service addressed to the party to whom it is being given at its

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address set forth above, or such other address as such party may have specified theretofore by notice delivered in accordance with this sentence. Any such notice shall be deemed to have been given and received on the date delivered or tendered for delivery during normal business hours as herein provided. A copy of any notice to Tenant shall also be sent to DLA Piper LLP (US), 33 Arch Street, Boston, MA 02110, Attn: Geoff Howell, Esq. and a copy of any notice to Mortgagee shall also be sent to Martha S. Faigen, Esq., 3 Center Plaza, Boston, MA 02108.

8. This Agreement may not be modified orally or in any manner than by an agreement in writing signed by the parties hereto or their respective successors in interest. This Agreement shall inure to the benefit of and be binding upon the parties hereto, their respective heirs, personal representatives, successors and assigns, and any purchaser or purchasers at foreclosure of the Property or any portion thereof, and their respective heirs, personal representatives, successors and assigns.

9. In the event the Mortgagee notifies Tenant of an Event of Default under the Loan and demands that Tenant pay its rent and all other sums due under the Lease to Mortgagee, Tenant agrees that it will honor such demand and pay its rent and all other sums due under the Lease to the Mortgagee. Landlord joins in this Agreement for the purpose of acknowledging that any payments to Mortgagee pursuant to this Section 9 shall be treated as payments of rent under the Lease, without any obligation of Tenant to verify the authenticity of such notice from Mortgagee.

10. Provided that there is no Event of Default under the Mortgage which is continuing, Mortgagee acknowledges and agrees that it shall permit the use of any insurance or condemnation proceeds by Landlord for the restoration of damage to the Premises or other Restoration Areas of the Property to the extent required by Article 15 of the Lease so long as Tenant is not in default under the Lease beyond applicable notice and cure periods.

[REMAINDER OF PAGE INTENTIONALLY BLANK]

EXHIBIT 13, PAGE 4

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IN WITNESS WHEREOF, the parties hereto have caused this Agreement to be duly executed as an instrument under seal as of the date first above written.

MORTGAGEE:  
EASTERN BANK

By: \_\_\_\_\_  
Name: \_\_\_\_\_  
Title: \_\_\_\_\_

TENANT:

uniQure, Inc.

By: \_\_\_\_\_  
Name: \_\_\_\_\_  
Title: \_\_\_\_\_

By: \_\_\_\_\_  
Name: \_\_\_\_\_  
Title: \_\_\_\_\_

LANDLORD

KING 113 HARTWELL LLC  
By: King Dickey LLC, its manager  
By: King Street Properties  
Investments LLC, its manager

By: \_\_\_\_\_

COMMONWEALTH OF MASSACHUSETTS

, SS.

On this date, , 2013, before me, the undersigned notary public, personally appeared -, the  
of King Street Properties Investments LLC, manager of King Dickey LLC, proved to me  
through satisfactory evidence of identification, which were to be the person whose name is signed on the preceding or  
attached document, and acknowledged to me that he signed it voluntarily for its stated purpose, in such capacity.

\_\_\_\_\_  
Notary Public  
My commission expires:

COMMONWEALTH OF MASSACHUSETTS

, SS.

On this date, , 2013, before me, the undersigned notary public, personally appeared , the  
of Eastern Bank, a corporation, proved to me through satisfactory evidence of identification, which were  
to be the person whose name is signed on the preceding or attached document, and acknowledged to me that he signed it  
voluntarily for its stated purpose.

\_\_\_\_\_  
Notary Public  
My commission expires:

COMMONWEALTH OF MASSACHUSETTS

, SS.

On this date, , 2013, before me, the undersigned notary public, personally appeared -, the  
of , a proved to me through satisfactory evidence of  
identification, which were to be the person whose name is signed on the preceding or attached document, and acknowledged  
to me that he signed it voluntarily for its stated purpose.

\_\_\_\_\_  
Notary Public  
My commission expires:

COMMONWEALTH OF MASSACHUSETTS

, SS.

On this date, , 2013, before me, the undersigned notary public, personally appeared -, the  
of , a proved to me through satisfactory evidence of  
identification, which were to be the person whose name is signed on the preceding or attached document, and acknowledged  
to me that he signed it voluntarily for its stated purpose.

\_\_\_\_\_  
Notary Public  
My commission expires:

King 113 Hartwell LLC, as Landlord under the Lease, and Mortgagor under the Mortgage, agrees for itself and its successors and assigns that:

The above agreement does not:

constitute a waiver by Mortgagee of any of its rights under the Mortgage or any of the other Loan documents; or

in any way release Mortgagor or Borrower from their obligations to comply with the terms, provisions, conditions, covenants and agreements and clauses of the  
Mortgage and other Loan documents;

The provisions of the Mortgage remain in full force and effect and must be complied with by Borrower; and

Following an Event of Default under the Mortgage, Tenant may pay all rent and other sums due under the Lease to Mortgagee as provided for above.

BORROWER/MORTGAGOR  
KING 113 HARTWELL LLC  
By: King Dickey LLC, its manager  
By: King Street Properties Investments LLC, its manager

By: \_\_\_\_\_  
Name: \_\_\_\_\_  
Title: \_\_\_\_\_

COMMONWEALTH OF MASSACHUSETTS

, ss.

On this date, \_\_\_\_\_, 2013, before me, the undersigned notary public, personally appeared \_\_\_\_\_, the \_\_\_\_\_ of King Street Properties Investments LLC, manager of King Dickey LLC, manager of King 113 Hartwell LLC, proved to me through satisfactory evidence of identification, which were \_\_\_\_\_ to be the person whose name is signed on the preceding or attached document, and acknowledged to me that he signed it voluntarily for its stated purpose in such capacity.

\_\_\_\_\_  
Notary Public  
My commission expires:

16 FEBRUARY 2012

Strictly Private & Confidential

Business Acquisition Agreement

between

Amsterdam Molecular Therapeutics (AMT) Holding N.V.  
as the Seller

uniQure B.V.  
as the Purchaser

Forbion Co-Investment II Coöperatief U.A.

Coöperatieve AAC LS U.A.

And

FORBION Co-Investment COÖPERATIEF U.A.

as the Investor

Amsterdam Molecular Therapeutics (AMT) B.V.

and

Amsterdam Molecular Therapeutics (AMT)IP B.V.  
as the Subsidiaries

relating to

the AMT human gene based therapy operations

**Simmons & Simmons**

Simmons & Simmons LLP PO Box 79023 1070 NB Claude Debussylaan 247 1082 MC Amsterdam The Netherlands  
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## SCHEDULES

schedule 1	Confidentiality and Standstill Agreement
schedule 2	Fairness Opinion
schedule 3	IPR and Further Assets and Liabilities
Schedule 4	Purchaser Warranties
Schedule 5	Seller Warranties
Schedule 6	Project Kairos Term Sheet
schedule 7	Press Release
Schedule 8	Notices

**THIS AGREEMENT** is dated and made on 16 February 2012

### **BETWEEN:**

- (1) **Amsterdam Molecular Therapeutics (AMT) Holding N.V.**, a public company (*naamloze vennootschap*), incorporated under the laws of the Netherlands with its registered office (*zetel*) in Amsterdam, and its business address at Meibergdreef 61, 1105 BA, Amsterdam, the Netherlands and registered with the Commercial Register (*Handelsregister*) of Amsterdam under number 33301321 (the “**Seller**”);
- (2) **uniQure B.V.**, a private company with limited liability (*besloten vennootschap met beperkte aansprakelijkheid*), incorporated under the laws of the Netherlands with its registered office in Amsterdam, and its business address at Gooimeer 2 35, 1411 DC Naarden, the Netherlands and registered with the Commercial Register of Gooi-, Eem- en Flevoland under number 54385229 (the “**Purchaser**”);
- (3) **Amsterdam Molecular Therapeutics (AMT) B.V.**, a private company with limited liability (*besloten vennootschap met beperkte aansprakelijkheid*), incorporated under the laws of the Netherlands with its registered office (*zetel*) in Amsterdam, and its business address at Meibergdreef 61, 1105 BA, Amsterdam, the Netherlands and registered with the Commercial Register (*Handelsregister*) of Amsterdam under number 34275365 (“**AMT BV**”);
- (4) **Amsterdam Molecular Therapeutics (AMT)IP B.V.**, a private company with limited liability (*besloten vennootschap met beperkte aansprakelijkheid*), incorporated under the laws of the Netherlands with its registered office (*zetel*) in Amsterdam, and its business address at Meibergdreef 61, 1105 BA, Amsterdam, the Netherlands and registered with the Commercial Register (*Handelsregister*) of Amsterdam under number 34275369 (“**AMT IP BV**”);
- (5) **Forbion Co-Investment II Coöperatief U.A.**, a cooperative (*coöperatie*), incorporated under the laws of the Netherlands with its registered office in Naarden, and its business address at Gooimeer 2 35, 1411 DC Naarden, the Netherlands and registered with the Commercial Register of Gooi-, Eem- en Flevoland under number 53951956, represented by its managing director **Forbion 1 CO II Management B.V.**, a private company with limited liability (*besloten vennootschap met beperkte aansprakelijkheid*), incorporated under the laws of the Netherlands with its registered office (*zetel*) in Naarden, and its business address at Gooimeer 2 35, 1411 DC Naarden, the Netherlands and registered with the Commercial Register of Gooi-, Eem- en Flevoland under number 53951956 (the “**New Investor**”);
- (6) **Coöperatieve AAC LS U.A.**, a cooperative (*coöperatie*), incorporated under the laws of the Netherlands with its registered office in Amsterdam, and its business address at Gooimeer 2 35, 1411 DC Naarden, the Netherlands and registered with the Commercial Register of Gooi-, Eem- en Flevoland under number 34256402, represented by its managing director **Forbion 1 Management B.V.**, a private company with limited liability (*besloten vennootschap met beperkte aansprakelijkheid*), incorporated under the laws of the Netherlands with its registered office (*zetel*) in Amsterdam, and its business address at Gooimeer 2 35, 1411 DC Naarden, the Netherlands and registered with the Commercial Register of Gooi-, Eem- en Flevoland under number 34249898 (the “**Existing Investor I**”);

- (7) **FORBION Co-Investment COÖPERATIEF U.A.**, a cooperative (*coöperatie*), incorporated under the laws of the Netherlands with its registered office in Naarden, and its business address at Gooimeer 2 35, 1411 DC Naarden, the Netherlands and registered with the Commercial Register of Gooi-, Eem- en Flevoland under number 32142360, represented by its managing director **Forbion 1 Management B.V.**, a private company with limited liability (*besloten vennootschap met beperkte aansprakelijkheid*), incorporated under the laws of the Netherlands with its registered office (*zetel*) in Amsterdam, and its business address at Gooimeer 2 35, 1411 DC Naarden, the Netherlands and registered with the Commercial Register of Gooi-, Eem- en Flevoland under number 342498 98 (the “Existing Investor II”);

each a “Party” and jointly referred to as the “Parties”. AMT BV and AMT IP BV jointly referred to as the “Subsidiaries” and the Seller and the Subsidiaries jointly referred to as the “Group”. The Existing Investor I and the Existing Investor II jointly referred to as the “Existing Investors”. The Existing Investors and the New Investor jointly referred to as the “Investor”.

#### **BACKGROUND:**

- (A) The Group is engaged in the development of human gene based therapies. The Seller acts as the holding company for the Subsidiaries and is listed on NYSE Euronext in Amsterdam.
- (B) Human gene-based therapies are intended to restore the natural function of the body as opposed to life long symptomatic treatment. These therapies are also very complex and it has taken years of innovative research to progress them to their current state where many of the traditional hurdles have been overcome. The Seller is a perceived leader in this field and the Executive Board (as defined hereafter) is of the view that the Group is well positioned to benefit from the anticipated growth of the area. It is against this background that the Seller had a regulatory setback with its lead product, which necessitated a change in strategy (pipeline focus).
- (C) On 21 October 2011 the Seller was informed that the European Medicine Agency’s Committee for Medical Products for Human Use (CHMP) maintained its earlier opinion that the Group’s lead product is not approvable at this time. The CHMP argues that the Group has not shown clinical benefit in a sufficient number of patients for a sufficiently long time. The CHMP admits that it sees the technology platform risks as marginal.
- (D) The fact that the CHMP issued a negative opinion regarding the Group’s lead product combined with the fact that the Group has very little cash available to continue the funding of its operations and the difficulties with attracting additional funding given the general economic climate and the Group’s prospects has led to concerns about the continuation of the Group’s business despite its strong position in the field.
- (E) Because of the CHMP’s negative opinion, the Seller redefined its strategy and decided to focus its development efforts and financial resources on its Hemophilia B, GDNF and AIP programs whilst suspending all investment in its lead product and the DMD program and implemented a reorganisation, which reorganisation included the redundancy of around 50% of the Group’s employees and a reduction of the Group’s operations (the “Reorganisation”).
- (F) With the aim of further reducing the cost base and to generate funds by means of milestone payments and royalties, the Seller has engaged in partnering discussions with

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various pharma companies, especially regarding its Hemophilia B program. At the same time, it continuously explored all options with regard to the Business (as defined herein) and its financing. The outcome of this exploration is that the Seller was informed repeatedly by its bankers that they are unable to assist to attract additional financing in the current circumstances, that the Seller has not been able to attract any interest from a significant number of specialist biotech funds and investors that it approached directly, and that there are no viable possibilities of a strategic party making a bid for the Business currently available.

- (G) Whilst the Seller anticipates that it may be possible to conclude a partnership on its Hemophilia B program in the first half of 2012, it is unlikely that it will be able to do so before its currently remaining cash is depleted. This means that to be able to continue its operations as a going concern, the Seller has an acute need to attract additional funding.
- (H) The Existing Investors hold a substantial share interest in the Seller and are also the holders of the Loan Notes (as defined herein). In view of the difficult position of the Group, the Investor and the Seller have been discussing the possibilities of a transaction that allows for the continuation of the Group’s current activities and the further advancement of its pipeline and which takes into account the justified interests of the Group’s stakeholders, including the shareholders of the Seller.
- (I) On 22 December 2011 the Seller and the Existing Investors entered into a confidentiality and standstill agreement, a copy of which is attached hereto as schedule 1 (the “Confidentiality and Standstill Agreement”).
- (J) Because of conflict of interest considerations at the Executive Board and Supervisory Board level a special committee consisting of Supervisory Board members Messrs. Ferdinand Verdonck (Chairman), Philippe Van Holle, Joseph Feczko and François Meyer (the “Special Committee”) was established which has been allocated the AMT decision-making regarding the possible Transaction (as defined here below) with the Purchaser and the Investor.
- (K) The Investor has been able to obtain funding commitments of €6.0 million in aggregate from a number of funds under its management, of which €5.0 million is committed by the New Investor that cannot invest in public companies, as a consequence of which taking the Business private is a prerequisite for this funding to be available.
- (L) The Special Committee has established that the Investor’s €6.0 million funding commitment offers the only significant funding opportunity that is available to the Group and that without this funding the Seller will likely have to apply for a moratorium of payment or file for bankruptcy on short notice. As a consequence and in comparison to an insolvency scenario, the funding opportunity the Investor offers provides an opportunity for the continuation of the Business and a chance to retain shareholder value and preserve jobs.
- (M) The Special Committee recognises that the Investor’s commitment to provide the aforementioned €6.0 million and to proceed with the Transaction is conditional to the Seller being able to obtain additional irrevocable financing commitments for the Business of at least €1.0 million in aggregate from an alternative investor, but believes that meeting this condition should be possible now that the Investor has expressed its willingness to further fund the Business.



- (N) The discussions between the Parties have resulted in an agreement relating to the sale and purchase of the Business and its financing as further described in, and subject to the terms and conditions of this Agreement (the “Transaction”).
- (O) Gene therapy is expected to advance medicine enormously in the area of many monogenic diseases and the Group has a leading position in this field. It is in this context that the Special Committee sees that the Transaction allows the Seller’s shareholders to maintain the value upside. It is the only financing transaction that the Special Committee believes to be able to execute on at the moment in the limited time frame available.
- (P) The Transaction requires the approval of the general meeting of shareholders of the Seller. On the basis that the Transaction is in the best interests of the Seller, its shareholders and the Business, the Special Committee supports the Transaction and shall recommend to the Extraordinary General Meeting (as defined hereafter) to approve the Transaction subject to the provisions of this Agreement.
- (Q) The Seller and the Special Committee have received a fairness opinion (the “Fairness Opinion”) dated 17 February 2012 from Ernst & Young LLP in connection with the Transaction and supporting the Special Committee’s recommendation of the Transaction, stating that the Transaction is fair and reasonable and in the interests of the Seller’s shareholders. A copy of the Fairness Opinion is attached as schedule 2.
- (R) The Parties have complied with or will comply with the provisions of the SER Merger Code 2000 (*SER-Fusiegedragsregels 2000*) and the Works Council Act (*Wet op de Ondernemingsraden*).

#### **AGREE AS FOLLOWS:**

##### **1. Interpretation**

- 1.1 Unless explicitly stated otherwise, the following terms shall have the following meaning (and grammatical variations of such terms shall have corresponding meanings):

“Accounts Receivable” means all the accounts receivable of the Seller relating to the Business;

“Administration” means all the administration of the Group relating to the Business, whether in electronic or physical form, including but not limited to ownership titles of assets, bought and sold ledgers, purchase and sales day books and purchase and sales invoices, management information records and other accounting books and records of the Group including tax records relating to the Business;

“Advance Distribution” has the meaning ascribed thereto in clause 13.2;

“Agreement” means this business acquisition agreement including the recitals and the Schedules hereto;

“Alternative Funding” shall mean unconditionally (except for customary closing conditions) and fully committed funding that would result in the Business being funded until at least the end of the calendar year 2012, which in the reasonable opinion of the Special Committee, acting in good faith, offers a better proposition for the future and continuity of the Company and its stakeholders than the Transaction;

“Alternative Offer” has the meaning ascribed thereto in clause 7.1;

“Alternative Offer Announcement” has the meaning ascribed thereto in clause 7.2;

“Alternative Offer Period” has the meaning ascribed thereto in clause 7.2;

“AMT BV” means Amsterdam Molecular Therapeutics (AMT) B.V.;

“AMT BV Shares” means all of the issued and outstanding shares in the share capital of AMT BV;

“AMT BV Seller Loan” means the intra Group loan agreement between the Seller (as lender) and AMT BV (as borrower) and all (existing and future) rights and obligations thereunder;

“AMT IP BV” means Amsterdam Molecular Therapeutics (AMT)IP B.V.;

“AMT IP BV Seller Loan” means the intra Group loan agreement between the Seller (as lender) and AMT IP BV (as borrower) and all (existing and future) rights and obligations thereunder;

“AMT IP BV Shares” means all of the issued and outstanding shares in the share capital of AMT IP BV;

“Business” means all activities of the Group, its operations and all its assets and liabilities, and includes the Sale Shares, the Loan Notes, the Contracts and Convertible Loan Note Agreement, the Seller Loans, the Intellectual Property Rights and the Further Assets and Liabilities, but excludes the Excluded Contracts;

“Business Day” means any day of the week (excluding Saturdays and Sundays) on which banks are open for business in the Netherlands;

“CIT Fiscal Unity Termination Date” has the meaning ascribed thereto in clause 16.3(B);

“Completion” means the consummation of the Transaction as contemplated in clause 10;

“Completion Date” means the date on which Completion shall occur and which shall be determined in accordance with clause 10.1;

“Conditions Precedent” has the meaning ascribed thereto in clause 8.1;

“Confidentiality and Standstill Agreement” has the meaning ascribed thereto recital (I);

“Contracts” means all contracts entered into by the Group relating to the Business and all existing and future rights and obligations thereunder as further set out in schedule 3 but excluding the Excluded Contracts;

“Convertible Loan Note Agreement” means the agreement constituting the issuance of loan notes dated 22 December 2009 and made by the Seller and Coöperatieve AAC LS U.A. and Forbion Co-Investment Coöperatief U.A. and all existing and future rights and obligations of the Seller thereunder;

“DCC” means the Dutch Civil Code (*Burgerlijk Wetboek*);

“Deed of Assignment” has the meaning ascribed thereto in clause 6.2;

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“Deed of Contribution” has the meaning ascribed thereto in clause 10;

“Distribution” has the meaning ascribed thereto in clause 13.2;

“Distribution Record Date” means the date that is the tenth Business Day following the date on which the dissolution of the Seller in accordance with clause 13.1 shall come into effect;

“Economic Ownership Transfer Agreement” means the agreement entered into on the date hereof by the Seller and the Subsidiaries pursuant to which the Seller agrees to transfer to the Subsidiaries the economic ownership of the NV-Business, subject to and on the terms and conditions set forth in such agreement;

“Employees” means all of the employees of the Group and includes the liabilities and obligations of the Seller towards or in relation to those employees, including all liabilities and obligations in relation to the Reorganisation;

“Encumbrance” means any right of pledge, mortgage, usufruct, retention of title or other security interest or other limited right (*beperkt recht*) whatsoever and any arrest, charge, attachment, option or lien or any similar concept that limits free and unrestricted title, under any applicable jurisdiction and any agreement or arrangement having the effect of providing security or priority;

“Excluded Contracts” means this Agreement, the listing agreement between the Seller and Euronext Amsterdam, the contract with the ENL agent and the payment agent, the liquidity provider agreement between the Seller and Kempen & Co N.V., the D&O Insurance policy of the Seller and any outstanding obligations under any stock option plan or other employee benefit plan of the Seller;

“Executive Board” means the executive board (*raad van bestuur*) of the Seller;

“Existing Investor I” means Coöperatieve AAC LS U.A., represented by its managing director Forbion 1 Management B.V.;

“Existing Investor II” means FORBION Co-Investment COÖPERATIEF U.A., represented by its managing director Forbion 1 Management B.V.;

“Existing Investors” means the Existing Investor I and the Existing Investor II;

“Extraordinary General Meeting” has the meaning ascribed thereto in clause 5.1;

“Fairness Opinion” has the meaning ascribed thereto in recital (Q);

“Final Distribution” has the meaning ascribed thereto in clause 13.2;

“Further Assets and Liabilities” means the Accounts Receivable, Administration, Intra-Group Trading Items, Employees and Contracts and any further assets and liabilities of the Group as further specified in schedule 3;

“Group” means the Seller and the Subsidiaries collectively;

“Guarantees” means any guarantees, indemnities, sureties, letters of comfort, joint and/or several liabilities or any other similar concept under applicable law pursuant to which a

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person or entity has or may become liable for the liabilities or obligations of another person or entity;

“Independent Tax Advisor” means an independent tax advisor or tax lawyer appointed by the Seller and Purchaser jointly with at least ten (10) years of experience as a practicing tax advisor or tax lawyer and being a member of the Dutch tax advisors association (*Nederlandse Orde van Belastingadviseurs*) or the Dutch association of tax lawyers (*Nederlandse Vereniging van Advocaat-Belastingkundigen*);

“Intellectual Property Rights” has the meaning ascribed thereto in schedule 3;

“Intra-Group Trading Items” means at any time, and from time to time, all amounts owed, outstanding or accrued in the ordinary course of trading between the Seller and the Subsidiaries in respect of intra group trading activity between them;

“Investor” means the New Investor and the Existing Investors;

“Loan Note 1” means the €700,000 5% convertible loan note due 2014 of the Seller dated 23 December 2009 and issued to and held by Coöperatieve AAC LS U.A. which is constituted by the Convertible Loan Note Agreement, and all the existing and future rights and obligations of the Seller thereunder;

“Loan Note 2” means the €4,300,000 5% convertible loan note due 2014 of the Seller dated 23 December 2009 and issued to and held by Forbion Co-Investment Coöperatief U.A. which is constituted by the Convertible Loan Note Agreement, and all the existing and future rights and obligations of the Seller thereunder;

“Loan Notes” means the Loan Note 1 and the Loan Note 2 collectively;

“Long Stop Date” means 12 May 2012;

“Matching Offer Right” has the meaning ascribed thereto in clause 7.2;

“New Investor” means Forbion Co-Investment II Coöperatief U.A., represented by its managing director Forbion 1 CO II Management B.V.;

“NV-Business” means all activities of the Seller, its operations and all its assets and liabilities, and includes the Accounts Receivable, the Contracts, the Intellectual Property Rights and the Further Assets and Liabilities (with the exception of the Administration), but excludes the Sale Shares, the Loan Notes, the Convertible Loan Note Agreement, the Seller Loans, the Excluded Contracts and the Economic Ownership Transfer Agreement;

“Opening Balance Sheet” means an opening balance sheet for Dutch corporate income tax purposes of AMT BV and AMT IP BV as at the CIT Fiscal Unity Termination Date as prescribed in article 13 of the Fiscal Unity Regulations 2003 (*Besluit fiscale eenheid 2003*) on a basis consistent with the Seller’s past practice (*bestendige gedragslijn*) and explanatory notes thereto;

“Party” or “Parties” means each of the Seller, the Purchaser, the Subsidiaries and the Investor individually or collectively;

“Purchaser” means uniQure B.V.;

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“Purchaser Warranties” means the representations and warranties (*garantieverklaringen*) made by the Purchaser to the Seller contained in Schedule 4;

“Recommendation” means the Special Committee’s recommendation to the Extraordinary General Meeting to approve the Transaction;

“Reorganisation” has the meaning ascribed thereto in recital (E);

“Sale Shares” means the AMT IP BV Shares and the AMT BV Shares;

“Schedule” means a schedule to this Agreement;

“Secondary Tax Liability” means any secondary liability for Tax for which the Seller is liable on the basis of article 39 and 43 of the Dutch Collection Tax Act 1990 (*Invoeringswet 1990*);

“Seller” means Amsterdam Molecular Therapeutics (AMT) Holding N.V.;

“Seller Loans” means the AMT BV Seller Loan and the AMT IP BV Seller Loan collectively;

“Seller’s Tax Matters” has the meaning ascribed thereto in clause 16.3(A);

“Seller Warranties” means the representations and warranties (*garantieverklaringen*) made by the Seller to the Purchaser contained in Schedule 5;

“Shareholders Circular” has the meaning ascribed thereto in clause 5.1;

“Signing Date” means 16 February 2012, being the date on which this Agreement is made and dated;

“Special Committee” has the meaning ascribed thereto in recital (J) and consists of Supervisory Board members Messrs. Ferdinand Verdonck (Chairman), Philippe Van Holle, Joseph Feczko and François Meyer;

“Subsidiaries” means AMT BV and AMT IP BV;

“Supervisory Board” means the supervisory board (*raad van commissarissen*) of the Seller;

“Surviving Provisions” means clause 1 (Interpretation), clause 9 (Break fee), clause 17 (Confidentiality/Public Announcement), clause 18 (Binding effect/Assignment), clause 19 (Partial invalidity), clause 20 (Entire agreement), clause 21 (Expenses), clause 22 (Dissolution and Annulment) clause 23 (Notices) and clause 24 (Governing law and jurisdiction) which shall survive any termination of this Agreement;

“Tax” or “Taxes” means any and all taxes and governmental and local duties, levies and fees of every kind, including without limitation corporate income tax, value added tax, property tax, import and excise duties, EU charges, wage and personal income taxes, transfer tax, capital gains tax, inheritance tax, environmental tax, capital tax and customs duties, retributions, social security contributions and premiums, and any interest, increases or penalties thereon;

“**Tax Authority**” means any taxing or other authority competent to impose any liability in respect of Taxes or responsible for the administration and/or collection of Taxes or enforcement of any law in relation to Taxes;

“**Tax Claim**” means any notice, demand, assessment, letter or other document by or on behalf of any Tax Authority resulting in a liability of the Seller or the Subsidiaries in respect of Tax;

“**Tax Liability**” means any actual liability of the Seller in respect of Tax attributable to the Business and the Subsidiaries or a Secondary Tax Liability;

“**Tax Relief**” includes any allowance, credit, deduction, exemption or set-off (including for the avoidance of doubt loss carry forwards) in respect of any Tax or relevant to the computation of any income, profits or gains for the purposes of any Tax, or any repayment of or saving of Tax (including any repayment supplement or interest in respect of Tax), and any reference to the use or set off of Tax Relief shall be construed accordingly and shall include use or set off in part and any reference to the loss or diminution of a Tax Relief shall include the absence, non-existence or cancellation of any such Tax Relief, or to such Tax Relief being available only in a reduced amount;

“**Transaction**” means the transaction contemplated by this Agreement and as further described in recital (N);

“**Transaction Resolutions**” has the meaning ascribed thereto in clause 5.4;

“**Trust Foundation**” has the meaning ascribed thereto in clause 4.1;

“**uniQure DR(s)**” has the meaning ascribed thereto in clause 2.2;

“**VAT Fiscal Unity Termination Date**” has the meaning ascribed thereto in clause 16.9; and

“**Working Hours**” means 9.30 a.m. to 5.30 p.m. on a Business Day.

1.2 In this Agreement, a reference to:

- (A) a “subsidiary” or “holding company” is to be construed in accordance with section 2:24(a) DCC;
- (B) a “group company” or “affiliate” is to be construed in accordance with section 2:24(b) DCC;
- (C) “material” or any similar expressions in this Agreement shall be construed in the context of the business of the Group taken as a whole;
- (D) singular words shall include the plural and *vice versa* and words in a particular gender shall include all genders, unless the context requires otherwise;
- (E) the word “include” or “including” are used to indicate that the matters listed are not a complete enumeration of all matters covered, unless the contrary is specifically stated;
- (F) the words “hereof”, “herein”, “hereto” and “hereunder” and words of similar import shall refer to this Agreement as a whole and not to any particular provision thereof;

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(G) a person includes a reference to a body corporate, association or partnership; and

(H) a clause or a schedule is a reference to a clause or schedule of the actual agreement.

1.3 In this Agreement, clause headings are inserted for convenience purposes only. They shall not affect the construction or interpretation of this Agreement.

1.4 In case of conflict between or inconsistency of the provisions of the actual agreement and the contents of the Schedules, the provisions of the actual agreement shall prevail.

1.5 The English language used in this Agreement intends to describe Dutch legal concepts only and the consequences of the use of this language in English law or any other law shall be disregarded. In case of conflict between Dutch legal concepts mentioned between brackets and/or in italics in this Agreement and the English translation thereof as used in this Agreement, the Dutch text, and its meaning thereof under Dutch law, will prevail.

## 2. **Sale of the Business**

2.1 Subject to the terms and conditions of this Agreement the Seller hereby agrees to contribute to the Purchaser and the Purchaser hereby agrees to accept from the Seller the Business.

2.2 Subject to the conclusion of the Economic Ownership Transfer Agreement and the execution of the Deed of Assignment in accordance with clause 6.2, the Business shall be transferred in accordance with clause 10 by way of a contribution in kind (*inbreng in natura*) on depositary receipts for (class B) ordinary shares in the capital of the Purchaser (the “**uniQure DRs**”), which uniQure DRs shall be issued to the Seller as consideration. The number of uniQure DRs to be issued to the Seller shall be equal to the number of shares in the Seller that shall be outstanding on the Completion Date.

## 3. **Funding of the Business**

3.1 The Investor commits to provide equity funding of €6.0 million to the Purchaser.

3.2 Pursuant to the Investor’s commitment to provide equity funding to the Purchaser, 9,771,986 (class A) ordinary shares in the capital of the Purchaser shall be issued to the Investor on or around the Completion Date at an issue price of €0.614 per share, being the mean closing share price of the Seller on NYSE Euronext in Amsterdam for the five Business Days prior to the Signing Date. In addition, the Seller’s debt under the Loan Notes as assumed by the

Purchaser pursuant to the Transaction shall be converted in 5,320,000 (class A) ordinary shares in the capital of the Purchaser issued to the Existing Investors, using a conversion price of €1.00 per share.

#### 4. **Organisation of the Purchaser**

- 4.1 The Investor shall procure that the Purchaser — a newly incorporated company without any liabilities — shall be structured and organized in accordance with the term sheet attached as Schedule 6 (Project Kairos Term Sheet) and that such structure and organisation shall have been implemented prior to Completion. This means inter alia that the Investor shall procure the incorporation of the trust foundation (the “Trust Foundation”) that shall issue the uniQure DRs and the preparation of the trust conditions pursuant where to the Trust Foundation shall hold the (class B) ordinary shares in the capital of the Purchaser on trust and of the shareholders agreement to be entered into between the shareholders of the Purchaser.

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- 4.2 All documentation to be prepared by the Investor and its advisers pursuant to clause 4.1 shall be prepared in close consultation with the Seller’s advisers and require the Seller’s approval, acting reasonably.

#### 5. **Extraordinary General Meeting and Recommendation**

- 5.1 The Transaction is subject to approval of the Seller’s general meeting of shareholders in accordance with section 2:107a DCC and the Seller’s articles of association. As soon as reasonably possible, the Seller shall convene an extraordinary general meeting of shareholders (the “Extraordinary General Meeting”) which shall be proposed to approve the Transaction and adopt the other Transaction Resolutions. The current target date of the Extraordinary General Meeting is 30 March 2012, which requires a convocation to be submitted no later than on 17 February 2012.
- 5.2 On the basis that the Transaction as contemplated by this Agreement is in the best interests of the Seller and its stakeholders and the Business, the Special Committee supports the Transaction and shall give the Recommendation.
- 5.3 The Recommendation may be modified or withdrawn by the Special Committee in the event an Alternative Offer having been made.
- 5.4 In addition to the proposal to approve the Transaction, the Extraordinary General Meeting shall be proposed to resolve:
- (A) subject to an Alternative Offer being made, to approve the disposal of the Business or a significant part thereof or the shares in capital of the Seller pursuant to an Alternative Offer, in view of section 2:107a of the DCC and section 20.1 of the Seller’s articles of association;
  - (B) subject to the execution of the Deed of Contribution, to dissolve and liquidate the Seller in accordance with section 47.1 of its articles of association with the members of the Executive Board becoming the liquidators of Seller’s dissolved property;
  - (C) subject to the execution of the Deed of Contribution, to approve the Advance Distribution;
  - (D) subject to the execution of the Deed of Contribution, to approve the Seller’s delisting from NYSE Euronext in Amsterdam;
  - (E) subject to the execution of the Deed of Contribution, to appoint AMT BV as the custodian (*bewaarder*) of the Seller’s books and records pursuant to section 2:24 of the Dutch Civil Code;
  - (F) to discharge (*decharge verlenen aan*) (i) the members of the Supervisory Board and (ii) the members of the Executive Board;
  - (G) to confirm in view of a (potential) conflict of interest between the Seller and members of the Executive Board that the shareholders meeting shall not make an appointment as referred to in clause 19.3 of the Seller’s articles of association; and

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- (H) to approve an amendment of the Seller’s articles of association to increase its authorised share capital to €3,200,000, divided in 80,000,000 shares with a nominal value of €0.04 each.

(the proposal to approve the Transaction and each of the other proposals to the Extraordinary General Meeting referred to in clause 5.4(B) through 5.4(H) collectively the “Transaction Resolutions”). The Extraordinary General Meeting shall also serve to discuss the measures to be taken in view of the Seller’s equity position in accordance with section 2:108a DCC.

- 5.5 The Existing Investors undertake with the Seller to support the Transaction and vote in favour of the Transaction Resolutions. The undertaking of the Existing Investors as per this clause 5.5 is unconditional and irrevocable and shall only terminate if this Agreement is terminated.
- 5.6 In relation to the Extraordinary General Meeting the Seller shall make a shareholders circular (the “Shareholders Circular”) available to its shareholders. The Shareholders Circular, which shall be in English only, shall include the Recommendation and the Fairness Opinion. The contents of the Shareholders Circular shall be agreed between the Seller and the Purchaser prior to its release.

#### 6. **Interim Period**

- 6.1 The Seller undertakes to the Purchaser that in the period from the Signing Date to the Completion Date, except with the approval of the Purchaser:
- (A) none of the Subsidiaries’ articles of association or other constitutional documents will be changed or altered;
  - (B) each of the Subsidiaries shall use its best endeavours to carry on its respective business in all material respects in the ordinary and usual course and consistent with past practice;

- (C) neither the Seller nor the Subsidiaries shall terminate or amend the employment agreements with any of the Employees other than with the prior written approval of the Purchaser;
- (D) no Subsidiary shall allot, issue, redeem or repurchase securities, loan capital (including shareholder loans and profit participation rights) or shall become a party to any agreement to do so; and
- (E) no dividend or other distribution or repayment of capital is, or shall be, paid or declared by the Seller or any of the Subsidiaries, other than the Distribution.
- 6.2 The Seller further undertakes to the Purchaser that on the date hereof the Seller and the Subsidiaries will enter into the Economic Ownership Transfer Agreement. In connection therewith the Seller undertakes that in the period from the Signing Date to the Completion Date the legal title to the NV-Business shall be transferred by the Seller to the Subsidiaries by means of the execution of a deed of assignment (the “Deed of Assignment”) to be mutually agreed by the Seller and the Purchaser acting in good faith. In order to implement such transfer in accordance with the Deed of Assignment, the Seller shall undertake all necessary actions, including but not limited to:

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- (A) by informing the debtors of the Accounts Receivable in writing that the Accounts Receivable have been assigned to the Purchaser;
- (B) by requesting the counterparties to the Contracts in writing for their co-operation to the transfer of contract to which the Seller is a party; and
- (C) to the extent not already referred to in this section 6.2, the proper fulfilment of applicable transfer requirements in respect of the Intellectual Property Rights and Further Assets and Liabilities owned and/or held by the Seller.
- 6.3 The Seller and the Purchaser hereby agree that in the period from the Signing Date to the Completion Date the Seller shall transfer the legal title to and economic ownership of the Seller Loans to the Subsidiaries, unless such transfer has materially adverse consequences for the Seller or the Subsidiaries; such determination to be made by the Seller and the Purchaser jointly acting reasonably.
7. **Alternative Offer and Alternative Funding**
- 7.1 For the purpose of this Agreement an “Alternative Offer” is an unconditional written *bona fide* offer by any third party for the Business or a significant part thereof or for the shares in the capital of the Seller, which may be a realistic and credible alternative to the Transaction for the Seller’s shareholders and other stakeholders.
- 7.2 If in the period starting on Signing Date and ending on the 16th day prior to the date of the Extraordinary Meeting (such period the “Alternative Offer Period”) the Seller receives one or more Alternative Offer(s), the Seller will as soon as possible, after having notified the Purchaser of such Alternative Offer, publicly announce that the Alternative Offer was made and the terms and conditions thereof (the “Alternative Offer Announcement”). The Purchaser may in its sole discretion amend its offer for the Business in such a manner that it matches or is superior to the Alternative Offer (the “Matching Offer Right”) for a period of five days following the Alternative Offer Announcement.
- 7.3 Before the Extraordinary General Meeting, the Special Committee shall indicate whether it intends, acting in good faith and taking into account the possible exercise of the Matching Offer Right by the Purchaser, to withdraw or materially modify its Recommendation.
- 7.4 If one or more Alternative Offer(s) is/are made and announced by way of the Alternative Offer Announcement the Extraordinary General Meeting shall be proposed to vote both on the Transaction as well as on the Alternative Offer(s) in accordance with and as set out in clause 5.4(A). In such case, the resolution by the Extraordinary General Meeting in relation to the Transaction, or as the case may be, an Alternative Offer that is approved by the Extraordinary General Meeting shall be pursued by the Seller, and if more than one transaction is approved by the Extraordinary General Meeting the Seller shall pursue the transaction approved with the highest majority of the votes cast at the Extraordinary General Meeting.
- 7.5 If the Seller pursues an Alternative Offer in accordance with clause 7.4, this Agreement may be terminated by each Party with immediate effect, by means of written notice to each other Party.
- 7.6 If the Seller achieves attracting Alternative Funding prior to Completion and pursues with such Alternative Funding, each Party may terminate this Agreement with immediate effect by means of written notice to each other Party.

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8. **Conditions Precedent**

- 8.1 The sale and purchase of the Business is subject to fulfilment on or before the Completion Date of each of the following conditions precedent (the “Condition(s) Precedent”):
- (A) the adoption of the Transaction Resolutions by the Extraordinary General Meeting and, in case one or more Alternative Offer is/are made, the adoption of the resolution(s) under clause 5.4(A), provided in such case that the procedure as set out in clause 7.4 has been followed by the Seller;
- (B) no application having been made to the Enterprise Division of the Amsterdam Court of Appeal pursuant to section 5:73 of the Act on financial supervision (*Wet op het financieel toezicht*) requesting that the Purchaser or any of its current shareholders make a public takeover bid;
- (C) no breach of the Seller Warranties or default under any of the terms and provision of this Agreement on the part of the Seller having occurred which has not been remedied to the reasonable satisfaction of Purchaser and which breach or default could reasonably be expected to constitute a material adverse effect on the Business and is of such material nature that it cannot be reasonably expected that the Purchaser continues with the Transaction and no facts or circumstances having occurred which could lead to such breach or default;

- (D) no breach of the Purchaser Warranties or default under any of the terms and provision of this Agreement on the part of the Purchaser having occurred which has not been remedied to the reasonable satisfaction of Seller and which breach or default is of such material nature that it cannot be reasonably expected that the Seller continues with the Transaction and no facts or circumstances having occurred which could lead to such breach or default;
  - (E) no action or proceeding by or before any court of law or arbitral tribunal or any governmental, provincial or municipal administrative body or authority or otherwise has been taken or instituted against any Party which may restrain, prohibit, invalidate or otherwise affect the transactions contemplated by this Agreement in any material respect;
  - (F) Euroclear Nederland having accepted the uniQure DRs for inclusion in and settlement through its book entry systems;
  - (G) SenterNovem's consent having been given to assign the agreement between the Seller and SenterNovem by the Seller to the Purchaser;
  - (H) irrevocable financing commitments being available to the Business in an amount of no less than EUR 1,000,000 (not including financing commitments extended by (funds managed by) the Investor); and
  - (I) no material adverse change has occurred in the profitability, financial or trading position or the prospects of the Group.
- 8.2 The Conditions Precedent under 8.1(A), 8.1(B), 8.1(E), 8.1(F) and 8.1(G) have been stipulated for the benefit of both the Seller and the Purchaser and may only be invoked or waived in writing by the Seller and the Purchaser acting jointly. The Conditions Precedent under, 8.1(C), 8.1(H) and 8.1(I) have been stipulated exclusively for the benefit of the Purchaser and may only be invoked or waived in writing by the Purchaser. The Condition

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Precedent under 8.1(D) has been stipulated exclusively for the benefit of the Seller and may only be invoked or waived in writing by the Seller.

- 8.3 The Seller and the Purchaser will, after execution of this Agreement, co-operate with each other to complete the applicable procedures under the applicable competition laws and regulations in the most expeditious manner and will further use their reasonable efforts to cause the fulfilment of the Conditions Precedent.
- 8.4 Should any Party become aware of anything which will or may prevent any of the Conditions Precedent from being satisfied it shall forthwith notify the same in writing to the other Parties.
- 8.5 In the event that any Condition Precedent has not been fulfilled or, to the extent permitted by law and this Agreement, has been waived by the Purchaser or the Seller (as the case may be) by the Long Stop Date, the Party or Parties for the benefit of which the relevant Condition Precedent has been stipulated may terminate this Agreement with immediate effect, by means of written notice to each other Party.
- 8.6 In the event this Agreement is terminated pursuant to clauses 7.5, 7.6 and 8.5, this Agreement shall cease to have effect, with the exception of the Surviving Provisions. In the event of such termination no Party shall be liable towards any other Party except for any liability towards any other Party in respect of a breach of this Agreement that took place prior to termination and except for the break fee that may be due pursuant to clause 9.
9. **Break fee**
- 9.1 A break fee is payable by the Seller to the Purchaser in the event this Agreement is terminated, except in the event of a termination of this Agreement as a result of the Condition Precedent under 8.1(D) being invoked, equal to compensate the Purchaser for all costs reasonably incurred (including, for the avoidance of doubt, legal fees) by the Purchaser and/or the Investor in connection with the Transaction, the break fee that may become payable pursuant to this clause 9.1 always being maximised at €200,000.
10. **Completion**
- 10.1 Completion shall take place at the offices of Stibbe N.V. in Amsterdam as soon as possible after the date on which the last Condition Precedent under clause 8.1 is satisfied or, where permitted, waived in writing by the relevant Party or Parties, save as the Seller and the Purchaser may agree otherwise in writing, which is expected to occur on or about five April 2012.
- 10.2 Following the conclusion of the Economic Ownership Transfer Agreement and the execution of the Deed of Assignment in the Interim Period in accordance with clause 6.2, the Business shall be transferred by the Seller to the Purchaser by means of the execution by one of the notaries of Stibbe N.V. of a notarial deed of contribution in kind (*akte van inbreng*) (the "Deed of Contribution") to be mutually agreed by the Seller and the Purchaser acting in good faith, pursuant to which the (class B) ordinary shares in the capital of the Purchaser underlying the uniQure DRs shall be issued to the Trust Foundation, with the Trust Foundation immediately subsequent issuing the uniQure DRs to the Seller. By means of the execution of the Deed of Contribution — as a consequence of which the Business shall be transferred — the Seller shall transfer to the Purchaser and the Purchaser shall accept from the Seller on the Completion Date:

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- (A) the Loan Notes and the Convertible Loan Note Agreement at a value equal to the nominal value and accrued interest and transfer of contract — the Parties agree and acknowledge that any and all rights under the Loan Notes and the Convertible Loan Note Agreement to convert any amount due under any Loan Note into shares in the Seller are terminated and extinguished as of the moment and by means of the execution of the Deed of Contribution;
- (B) the Administration (also by giving the Purchaser possession (*bezitsverschaffing*) thereto and, if necessary, notification to third parties holding (parts of) the Administration for the Group);
- (C) the Seller Loans at a value equal to the nominal value and accrued interest, in the event the Seller has not transferred the legal title to and economic ownership of these Seller Loans to the Subsidiaries between the Signing Date and the Completion Date, due to the existence of materially adverse consequences for the Seller or the Subsidiaries; and

- (D) the Sale Shares.
- 10.3 To transfer the Business, on the Completion Date the Purchaser shall assume from the Seller and the Seller shall transfer to the Purchaser the Guarantees, whilst the Seller shall forthwith upon request of the Purchaser request the creditors of the Guarantees in writing for their consent to the transfer of the Guarantees to the Purchaser.
- 10.4 To the extent any Employees are employed by Seller and not by the Subsidiaries and have not been transferred to the Purchaser on the Completion Date as a result of the transfer of the Sale Shares such Employees (and the rights and obligations under the employment agreements with such Employees in force at the Completion Date) will be transferred to the Purchaser by operation of law as a result of transfer of undertaking (*overgang van onderneming*) in accordance with section 7:663 *et seq.* DCC.
- 10.5 The Purchaser shall procure the due compliance with section 2:204b DCC in relation to the contribution in kind by means of the Deed of Contribution, including the preparation of the description of the contribution and the preparation of the auditor's statement.
11. **Co-operation following the Completion Date**
- 11.1 The Parties shall do all such further acts and execute all such further documents as shall be necessary to fully effect the transfer of any part of the Business following Completion. The Parties shall take all necessary steps and cooperate fully to ensure that the Purchaser acquires (or assumes, as the case may be) the Business and the Parties shall at first request execute such documents in such form as may be agreed between the Seller and the Purchaser and take such other steps as are necessary or appropriate for vesting in the Purchaser all of the Seller's rights and interests in the Business.
- 11.2 Insofar as any of the Contracts or any other (asset or liability that is) part of the Business cannot be effectively transferred to the Purchaser without the consent of a third party or except by an agreement of novation on the Completion Date and the Parties have nevertheless proceeded with the Completion, then:

- (A) the Purchaser acting on behalf of the Seller shall use all reasonable endeavours to obtain such consent or to procure a novation as soon as is reasonably possible following the Completion Date;
- (B) unless and until such consent is obtained or novation has taken place the Purchaser shall for its own account perform on behalf of the Seller (but at the Purchaser's expense) all the obligations of the Seller existing under such Contracts or (other asset or liability that is) part of the Business and the Seller will observe and comply with Purchaser's reasonable instructions in relation to the same;
- (C) any and all benefits under such Contracts or other (asset or liability that is) part of the Business shall be for the account of the Purchaser, and if received by the Seller, shall be forthwith paid on to the Purchaser by the Seller; and
- (D) the Purchaser and the Seller may decide in mutual consent to terminate certain Contracts.
- 11.3 The Purchaser shall procure, with effect from the Completion Date, the release of the Seller from any (joint and/or several) Guarantees and other liabilities given by, assumed by or binding upon the Seller in relation to any of the liabilities of the Group. The Purchaser shall indemnify and hold the Seller harmless and, against all amounts to be paid by it to any such (joint and several) Guarantees and other liabilities with respect to the Business (whether relating to the period before or after Completion).
12. **Post-Completion undertakings**
- 12.1 The objective of the Transaction is that at Completion the entire Business and all its historic, actual and future assets and liabilities are transferred to the Purchaser, effectively transforming the Seller in a listed shell company with the uniQure DRs as its single asset and without any liabilities and that the uniQure DRs shall be subsequently available for distribution to the Seller's shareholders by way of the Distribution as further set out in clause 13. In view of this objective:
- (A) the Purchaser will, provided that Completion has taken place, indemnify the Seller and hold the Seller fully harmless (*vrijwaren en schadeloosstellen*) against all claims, liabilities, losses, costs (including the costs of legal and other advisers), damages, charges, expenses, proceedings and actions relating or attributable to the Business, whether relating to the period before or after Completion;
- (B) the Purchaser shall compensate the Seller on a euro for euro basis for all costs associated to its operations as from Completion until the moment that the liquidation terminates and the Seller ceases to exist as further set out in clause 13, including but not limited to the costs and fees incurred and to be incurred pursuant to the Excluded Contracts, the listing, the preparation of the financial statements for the financial year 2011 and other financial reports, the 2012 annual general meeting and the Tax arrangements set out in clause 16;
- (C) the Purchaser shall at no cost make the services and assistance of the Employees available to the Seller to the extent reasonably required or desired in relation to the Seller's operations as from Completion until the moment that the liquidation terminates and the Seller ceases to exist, including but not limited to services and assistance in relation to prepare financial statements and other financial reporting, required disclosures, compliance matters and matters associated to the dissolution and liquidation of the Seller as further set out in clause 13.

- 12.2 The Purchaser shall, and shall procure that the Subsidiaries shall, promptly and timely provide copies taken from the Administration and all further information and documentation relating to the Business that the Seller may reasonably require for the purpose of preparing its financial statements or otherwise request.
- 12.3 Except to the extent that liability arises from the gross negligence (*grove nalatigheid*) or wilful misconduct (*opzet*) of the relevant person, the Purchaser (by way of irrevocable third party stipulation for no consideration) shall indemnify each member of the Special Committee, the Executive Board and the



Supervisory Board and hold them fully harmless (*vrijwaren en schadeloosstellen*) against all claims, liabilities, losses, costs (including the costs of legal and other advisers), damages, charges, expenses, proceedings and actions relating or attributable to the Transaction, including for the avoidance of doubt the dissolution, the Distribution and delisting as set out in clause 13, and shall furthermore procure that appropriate D&O insurance for the benefit of the Special Committee and the members of the Executive Board and Supervisory Board shall be available and remain available at the Purchaser's costs from Completion until the moment that the liquidation terminates and the Seller ceases to exist.

13. **Dissolution, Distribution and delisting**

- 13.1 If and to the extent the Extraordinary General Meeting adopts the resolution to approve the Transaction and subject to the execution of the Deed of Contribution, the Seller shall be dissolved in accordance with section 47.1 of the Seller's articles of association and section 2:23a et seq. DCC with the Executive Board members becoming the liquidators of the Seller's dissolved property.
- 13.2 Because the Seller's dissolved property will consist of the uniQure DRs issued to the Seller in consideration for the Business without any known financial liabilities, and the financial condition is expected to justify the same, it is intended that the liquidators shortly after the Completion Date make an advance liquidation distribution to the Seller's shareholders by distributing and allocating to each such shareholder one uniQure DR for each share in the Seller held on the Distribution Record Date (the "Advance Distribution"). Should any liquidation surplus remain after the Advance Distribution, the liquidators will make a final liquidation distribution to the Seller's shareholders of such remaining liquidation surplus as soon as reasonably possible after the liquidation accounts and plan of distribution have become final (such final distribution, if any, the "Final Distribution", the Advance Distribution and the Final Distribution (if any) also, the "Distribution").
- 13.3 The Distribution, to the extent it concerns the distribution of the uniQure DRs, shall be an allocation of such securities where there is no element of choice on the part of the recipient, including no right to repudiate the allocation. Consequently, the Distribution is no "offer of securities to the public" in the meaning of Chapter 5.1 of the Financial Supervision Act (*Wet op het financieel toezicht*) and article 2.1 (d) of the Prospectus Directive (2003/71/EC - 2010/73/EU). As a result the Distribution does not require the publication of a prospectus, nor does any other element of the Transaction.
- 13.4 The Purchaser shall procure that upon issue the uniQure DRs shall be included in the Euroclear Nederland operated book entry systems, enabling the Distribution of the uniQure DRs being settled through such book entry system, resulting in one uniQure DR being credited in the securities account of each of the Seller's shareholder for each share in the Seller held on the Distribution Record Date.

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- 13.5 When the liquidation ends the Seller shall cease to exist, which shall effectively also result in the Seller's delisting, to the extent the delisting cannot be achieved at an earlier date in consultation with Euronext Amsterdam and subject to such conditions as Euronext Amsterdam may propose.

14. **Seller Warranties**

- 14.1 Subject to Completion and the provisions of this clause 14, the Seller represents and warrants to the Purchaser that each of the Seller Warranties is accurate on the Signing Date and shall be accurate on the Completion Date.
- 14.2 The Purchaser acknowledges that the Seller Warranties are the only representations, warranties or other assurances of any kind on which the Purchaser may rely in entering into this Agreement. No statement made or information provided by or on behalf of the Seller, the Subsidiaries or any of their affiliates or advisors can be regarded as a representation, warranty or other assurance of any kind.
- 14.3 Subject to the limitations of clauses 14.4 through 14.6, in the event of a breach of any Seller Warranty, the Seller shall, at the direction of the Purchaser, pay to the Purchaser or any other designated person, at the option of the Purchaser, an amount equal to:
- (A) the amount necessary to place the Purchaser and the Subsidiaries in the position in which each of them would have been if the relevant breach had not occurred; or
- (B) the actual amount of damage (*schade*) suffered or incurred by the Purchaser.
- 14.4 The aggregate liability of the Seller pursuant to this clause 14 shall not exceed €1,000,000.
- 14.5 The Purchaser's right to claim compensation for damage for a breach of one or more of the Seller Warranties lapses on the earlier of (i) six months after the Signing Date and (ii) the moment on which the Seller dissolves, unless prior to the relevant expiry date valid notice of a valid claim has been given by the Purchaser to the Seller in accordance with clause 14.6.
- 14.6 If the Purchaser becomes aware of anything that constitutes or may constitute a breach of or may be inconsistent with the Seller Warranties, the Purchaser will give immediate written notice to the Seller of all the relevant facts known at the time to the Purchaser. The Purchaser shall have no right to claim damages if the Purchaser has not initiated legal proceedings against the Seller within two months after the lapse of the relevant limitation period referred to clause 14.3.

15. **Purchaser Warranties**

- 15.1 The Purchaser represents and warrants to the Seller that each of the Purchaser Warranties is accurate on the Signing Date and shall be accurate on the Completion Date.
- 15.2 The Purchaser shall be liable to the Seller for and shall indemnify and hold the Seller harmless against all losses and damages (including, without limitation, all reasonably incurred legal fees and costs of litigation) suffered by the Seller as a result of a breach of one or more of the Purchaser Warranties.

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16. **Additional Tax arrangements**

#### Tax indemnities

- 16.1 The Purchaser will indemnify the Seller and hold the Seller fully harmless against all actions, claims, costs, interest and expenses (including fees of legal and other advisers), provided that these costs and expenses have been reasonably incurred, in relation to:
- (A) any actual liability in respect of Tax for which the Seller is liable as a result of any event occurring before or on Completion Date or in respect of any profits earned or revenues realised before or on the Completion Date (excluding for the avoidance of doubt any Dutch dividend withholding tax), which are attributable to the Business and/or the Subsidiaries;
  - (B) any Secondary Tax Liability.

#### Due date for payment

- 16.2 A payment to be made by the Purchaser under clause 16.1(A) or 16.1(B), shall be made within ten Business Days after the earlier of (i) the date on which the Seller and the Purchaser have agreed jointly (x) not to file an appeal against a Tax assessment or similar instrument which constitutes a payment obligation, or (y) not to file a further appeal against a decision with respect to a Tax Liability; (ii) the date on which a final decision is made on appeal with respect to a Tax Liability against which no further appeal is possible or (iii) the date on which the Seller, with the prior consent of the Purchaser, in the context of a Tax Liability has made a payment to any Tax Authority on the basis of a Tax assessment or similar instrument which constitutes a payment obligation which cannot be suspended or is paid to avoid interest or penalties.

#### *Procedural arrangements*

#### Seller's Tax Matters

- 16.3 The Parties agree that:
- (A) the Seller or such professional advisers as the Seller may determine shall, with due observance of clause 16.3(E) and 16.3(F), conduct the preparation, submission and negotiation of all returns and computations, the preparation and submission of all correspondence relating to such returns and computations and the agreement of all matters relevant to the Tax position of the CIT Fiscal Unity, the Seller and, for any period ending on or before the Completion Date, the Subsidiaries (the "Seller's Tax Matters");
  - (B) the Seller and Purchaser will agree jointly on the date of termination of the Dutch fiscal unity for Dutch corporate income tax purposes within the meaning of article 15 Dutch Corporate Income Tax Act 1969 (*Wet op de vennootschapsbelasting 1969*) (the "CIT Fiscal Unity Termination Date") and shall not take any positions in their Tax affairs inconsistent therewith. Within three months following the Completion Date, the Seller shall present the Purchaser with an Opening Balance Sheet. The Purchaser is entitled to dispute the Opening Balance Sheet in writing within 10 Business Days after delivery thereof. In case the Seller and the Purchaser are not able to remove the objections of the Purchaser amicably within 5 Business Days, an Independent Tax Advisor shall decide on the objection and to the extent necessary revise the Opening Balance Sheet.
  - (C) Any Tax Relief attributable to the Business and/or the Subsidiaries shall be transferred by the Seller to the Purchaser or the Subsidiaries to the maximum extent possible, such that it will be available to the Purchaser or the Subsidiaries after Completion. In accordance with article 15af of the Dutch Corporate Income Tax Act, the Seller and the Subsidiaries shall, together with the CIT Tax return for the period during which the Fiscal Unity Termination Date occurs, submit a request to the Tax authorities to transfer to the Subsidiaries the remaining losses available for loss carry forward attributable to the Subsidiaries.
  - (D) the Purchaser shall, and shall procure that the Subsidiaries shall co-operate fully with the Seller for the purpose of the Seller's Tax Matters and the compilation of the Opening Balance Sheet and will cause the Subsidiaries to provide the Seller promptly and timely with any information received by the Purchaser or the Subsidiaries, which is relevant to the Seller's Tax Matters and with any information as deemed necessary by the Seller to compile the Opening Balance Sheet and upon the Seller's first request with all reasonable information, documentation and assistance requested with reasonable priority and will provide Seller and its advisers access to the Subsidiaries' books and records during reasonable business hours, to the extent reasonably required to assess the Tax position of the Seller;
  - (E) the Seller shall keep the Purchaser fully informed of the progress of all matters relating to the Seller's Tax Matters and shall provide the Purchaser with copies of all written correspondence with any Tax Authority if and to the extent relevant for the Tax affairs of the Seller;
  - (F) the Seller shall not, without the written consent of the Purchaser, file returns, make claims, elections or statements or enter into an agreement or settlement with any Tax Authority in respect of the Seller's Tax Matters if such a return, claim, election, statement or such an agreement or settlement could reasonably lead to adverse Tax consequences for the Purchaser.
  - (G) the Subsidiaries shall, subject to clause 16.3(E) and 16.3(F) being fulfilled, immediately authorise, sign and submit to the relevant Tax Authority such returns and other ancillary information, accounts, statements and reports relating to a relevant Tax period and make such claims and elections and give such consents to comply with all procedural requirements in respect of the making or giving of such returns, ancillary information, accounts, statements and reports or such claims, elections or consents as the Seller may direct in writing to the extent that they relate to the Seller's Tax Matters;
  - (H) if the Seller directs the Subsidiaries to make a payment for Wage Taxes, social securities and VAT to any Tax Authority in respect of any matter over which the Seller has conduct, the Subsidiaries shall make the payment to the relevant Tax Authority within two Business Days of the Subsidiaries receiving the written instruction from the Seller;
  - (I) The Purchaser nor the Subsidiaries shall take any positions in their Tax affairs inconsistent with the Opening Balance Sheet (as revised by the Independent Tax Advisor).

- 16.4 The Seller shall up to the Completion Date continue its Tax affairs and the Tax affairs of the Subsidiaries in a manner consistent with past practice (*bestendige gedragslijn*), and:

- (A) the Seller shall not deviate and shall cause the Subsidiaries not to deviate from the principles consistently applied and the courses of action consistently followed in respect of (i) the determination of the profit for Tax purposes (*fiscale winstbepaling*); and (ii) the valuation of the assets and liabilities for Tax purposes; and
- (B) the Seller shall ensure that no decisions or any other actions are taken by it or the Subsidiaries which may prejudice or otherwise cause an adverse change in its Tax position or the Tax position of the Subsidiaries,

unless required by applicable Tax law or written permission has been granted by the Purchaser

The terms ‘consistently applied principles’ and ‘course of action consistently followed’ in the previous sentence, *inter alia*, relate to the depreciation schedule, transfer pricing, and the moment revenue, income, gain, loss, cost and expenditures are recognised for Tax and accounting purposes.

#### Purchaser’s Tax Matters

- 16.5 With effect from Completion, the Purchaser and its advisers shall have sole conduct of all Tax affairs of the Subsidiaries other than the Seller’s Tax Matters.

#### Conduct of Tax Claims

- 16.6 Subject to Completion, if the Seller receives any Tax Claim, the Seller shall give or procure that notice in writing is given to the Purchaser as soon as is reasonably practicable.
- 16.7 The Purchaser shall be entitled at its own expense to resist the Tax Claim in the name of the Seller or as the case may be the Subsidiaries and to have the conduct of any appeal, dispute, compromise or defence of the Tax Claim and of any incidental negotiations and the Seller will give and procure the Subsidiaries to give the Purchaser all reasonable co-operation, access and assistance for the purposes of considering and resisting the Tax Claim.
- 16.8 If the Purchaser does not elect to resist that Tax Claim in the name of the Seller, the Seller shall give the Purchaser drafts of all communications it intends to make in relation to the Tax Claim at least five Business Days before the communication is made, shall make such amendments as the Purchaser shall request unless, in the reasonable opinion of the Seller, such amendments may adversely affect the tax position of the Seller, before it makes such communication and shall promptly provide the Purchaser with copies of all correspondence relating to the Tax Claim.

#### VAT

- 16.9 The Seller shall cause the request referred to in article 43, paragraph 1 of the Dutch Collection Tax Act 1990 (*Invorderingswet 1990*) to be submitted to the competent Tax Authorities on the Completion Date in order to effectuate the termination of the Dutch fiscal unity for Dutch VAT purposes within the meaning of article 7 paragraph 4 Dutch VAT Act 1968 (*Wet op de omzetbelasting 1968*) between the Seller and the Subsidiaries as from the Completion Date (the “VAT Fiscal Unity Termination Date”).

- 16.10 The Seller and the Purchaser shall determine jointly whether there are registrations for VAT purposes of the Seller’s Group outside The Netherlands that have to be terminated and shall cause the termination of such registration(s). The Purchaser shall in its sole discretion determine whether to apply for new registrations for VAT purposes of the Purchaser’s Group, as from the Completion Date.

#### Confidentiality

- 16.11 Any information obtained by a Party from another Party in connection with any Tax matters to which this clause 16 applies shall be kept confidential, except as otherwise provided in this Agreement and except as may be otherwise necessary in connection with the filing of tax returns or claims for refund or in conducting an audit or other tax proceedings relating to the disclosing party or to comply with any statutory requirements.

#### Miscellaneous

- 16.12 The provisions of this clause 16 exhaustively constitute the obligations of the Seller relating to Tax.

#### **Confidentiality/Public Announcement**

- 17.1 Except to the extent required by law (including the SER Merger Code 2000) or applicable stock exchange regulations and in case of such requirement only after prior consultation with the other Party (to the extent reasonably practicable), the Parties will not disclose the content of this Agreement and the discussions and negotiations to any third party.
- 17.2 The Parties will consult and agree in advance with each other on the timing and tenor of any public announcement with respect to the Transaction, provided however that no Party shall be required to abstain from any public announcement that that is required by law or applicable stock exchange regulations.
- 17.3 Nothing in this clause 17 shall affect the rights and obligations of the relevant Parties under the Confidentiality and Standstill Agreement.
- 17.4 Immediately after the execution of this Agreement, the Seller shall issue the press release in the agreed form attached as schedule 7.

#### **Binding Effect/Assignment**

- 18.1 All the terms, provisions, warranties, covenants and conditions of this Agreement shall only be binding upon and inure to the benefit of and be enforceable by the Parties after this Agreement has been signed by the Parties.
- 18.2 Except as expressly provided otherwise, this Agreement does not contain any third-party stipulation (*derdenbeding*).
- 18.3 This Agreement and any rights and obligations of the Parties hereto may not be transferred, assigned or delegated by any Party to a third party without the prior written consent of the Seller.

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19. **Partial Invalidity**

- 19.1 In the event that one or more clauses of this Agreement or of the Schedules are established to be non-binding, the other clauses of this Agreement and of the Schedules will continue to be effective. The Parties are obliged to replace the non-binding clauses with other clauses that are binding, in such a way that the new clauses differ as little as possible from the non-binding clauses, taking into account the object and the purpose of this Agreement.

20. **Entire Agreement; counterparts**

- 20.1 This Agreement including the Schedules thereto contains all arrangements, which the Parties have made on the subject. They shall replace and supersede all other (previous) arrangements and agreements, which the Parties have made or have entered into on the subject. This Agreement can only be altered or modified by means of a document signed by the Parties.
- 20.2 This Agreement may be executed in any number of counterparts by the Parties to it, each of which shall be deemed an original, but all of which together shall constitute one and the same instrument.

21. **Expenses**

- 21.1 Each of the Parties hereto shall pay its own expenses incurred or to be incurred in connection with the negotiation and preparation of this Agreement and the transactions contemplated thereby, except for the expenses incurred or to be incurred by the Investor, which expenses shall be borne by the Purchaser.

22. **Dissolution and Annulment**

- 22.1 Each of the Parties hereby waives the right, and each of the Parties accepts the same, to cancel (*opzeggen*), to dissolve or bring an action to dissolve this Agreement (*ontbinding*) and/or to annul or bring an action to annul this Agreement (*vernietiging*) or alter this Agreement on the basis of unforeseen circumstances (*onvoorziene omstandigheden*) or suspend (*opschorten*) any of the obligations assumed hereunder as from the moment of its execution.
- 22.2 Each of the Parties hereby further waives, and each of the Parties accepts the same for the other Parties, the applicability of title 1 of Book 7 DCC.

23. **Notices**

- 23.1 All announcements or notices to the Parties will be done in writing and delivered to the relevant Party at its address specified in Schedule 8 as long as a Party does not give notice to the other Parties of any other address.
- 23.2 Subject to clause 23.3, in the absence of evidence of earlier receipt, any notice or other communication given under this Agreement shall be deemed to have been duly given as follows:
- (A) if delivered personally or by courier, on delivery;
- (B) if sent by registered mail, two clear Business Days after the date of posting; and

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- (C) if sent by email, at the time of completion of transmission provided that the recipient of the email acknowledges receipt of such email.
- 23.3 Any notice given under this Agreement outside Working Hours in the place to which it is addressed shall be deemed not to have been given until the start of the next period of Working Hours in such place.

24. **Governing law and Jurisdiction**

- 24.1 This Agreement and any contractual and non contractual obligations arising there from shall be governed exclusively by Dutch law.
- 24.2 All disputes arising out of or in connection with this Agreement or further agreements resulting thereof shall be settled by arbitration in accordance with the rules of the Netherlands Arbitration Institute (*Nederlands Arbitrage Instituut*). The Arbitral Panel will consist of three arbitrators and the proceedings will be conducted in English (unless the Parties agree otherwise) in Amsterdam, The Netherlands. The Arbitral Panel will rule in accordance with provisions of Dutch law.

[signature pages follow ]

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/s/Jörn Aldag  
By: Mr. J. Aldag  
Title: Chief Executive Officer

/s/PJ Morgan  
By: Mr. P.J. Morgan  
Title: Chief Financial Officer

uniQure B.V.

uniQure B.V.

/s/H.A. Slootweg  
By: Forbion 1 Co II Management B.V.  
Title: Director  
By: H.A. Slootweg  
Title: Director

/s/M.A. van Osch  
By: Forbion 1 Co II Management B.V.  
Title: Director  
By: M.A. van Osch  
Title: Director

Amsterdam Molecular Therapeutics (AMT) B.V.

Amsterdam Molecular Therapeutics (AMT) B.V.

/s/ Jörn Aldag  
By: Amsterdam Molecular Therapeutics (AMT) Holding N.V.  
Title: Director  
By: Mr. J. Aldag  
Title: Chief Executive Officer

/s/PJ Morgan  
By: Amsterdam Molecular Therapeutics (AMT) Holding N.V.  
Title: Director  
By: Mr. P.J. Morgan  
Title: Chief Financial Officer

Amsterdam Molecular Therapeutics (AMT)IP B.V.

Amsterdam Molecular Therapeutics (AMT)IP B.V.

/s/ Jörn Aldag  
By: Amsterdam Molecular Therapeutics (AMT) Holding N.V.  
Title: Director  
By: Mr. J. Aldag  
Title: Chief Executive Officer

/s/PJ Morgan  
By: Amsterdam Molecular Therapeutics (AMT) Holding N.V.  
Title: Director  
By: Mr. P.J. Morgan  
Title: Chief Financial Officer

Forbion Co-Investment II Coöperatief U.A.

Forbion Co-Investment II Coöperatief U.A.

/s/H.A. Slootweg  
By: Forbion 1 Co II Management B.V.  
Title: Director  
By: H.A. Slootweg  
Title: Director

/s/M.A. van Osch  
By: Forbion 1 Co II Management B.V.  
Title: Director  
By: M.A. van Osch  
Title: director

Coöperatieve AAC LS U.A.

Coöperatieve AAC LS U.A.

/s/H.A. Slootweg  
By: Forbion 1 Management B.V.  
Title: Director  
By: H.A. Slootweg  
Title: Director

/s/M.A. van Osch  
By: Forbion 1 Management B.V.  
Title: Director  
By: M.A. van Osch  
Title: director

FORBION Co-Investment COÖPERATIEF U.A.

FORBION Co-Investment COÖPERATIEF U.A.

/s/H.A. Slootweg  
By: Forbion 1 Management B.V.  
Title: Director  
By: H.A. Slootweg  
Title: Director

/s/M.A. van Osch  
By: Forbion 1 Management B.V.  
Title: Director  
By: M.A. van Osch  
Title: director

22 December 2011

Confidentiality and Standstill Agreement

between

**Amsterdam Molecular Therapeutics (AMT) Holding N.V.**

and

**Forbion I Management B.V.**

relating to Project Kairos

Simmons & Simmons

Simmons & Simmons LLP PO Box 79023 1070 NB Claude Debussylaan 247 1082 MC Amsterdam The Netherlands  
T +31 20 722 2500 F +31 20 722 2599

Simmons & Simmons LLP is a limited liability partnership registered with the Registrar of Companies for England & Wales with number OC352713 and with its registered office at CityPoint, One Ropemaker Street, London EC2Y 9SS, United Kingdom, as well as registered with the trade register kept with the Chamber of Commerce in Amsterdam, the Netherlands, with number 51088282 with registered office at the above address. The word “partner” refers to a member of Simmons & Simmons LLP or an employee or consultant with equivalent standing and qualifications. A list of members and other partners and their professional qualifications is available for inspection at all our offices. Our terms of business, which contain a limitation of our liability, apply to all our services.

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## **CONFIDENTIALITY AND STANDSTILL AGREEMENT**

### **BETWEEN:**

- [(1) **Amsterdam Molecular Therapeutics (AMT) Holding N.V.** a public company (*naamloze vennootschap*), incorporated under the laws of the Netherlands with its registered office (*zetel*) in Amsterdam, and its business address at Meibergdreef 61,105 BA, Amsterdam Zuidoost and registered with the Dutch Commercial Register (*Handelsregister*) of Amsterdam under number 33301321 (“Company”); and
- (2) **Forbion I Management B.V.**, a private company with limited liability (*besloten vennootschap met beperkte aansprakelijkheid*), incorporated under the laws of the Netherlands with its registered office (*zetel*) in Amsterdam, and its business address at Gooimeer 2-35, 1411 DC Naarden and registered with the Dutch Commercial Register (*Handelsregister*) of Amsterdam under number 34249888 (the “Recipient”).

The Company and the Recipient, jointly the “Parties”.

### **WHEREAS:**

- (A) the Recipient is interested in investigating the possibilities of pursuing a transaction (“Transaction”) involving certain or all assets of the Company or an alternative transaction regarding the Company and its subsidiaries (all together, including any affiliates, hereinafter referred to as the “Group”), in the context of which the Recipient may receive certain Information (as defined herein);
- (B) in consideration of the Company’s willingness to make Information available to the Recipient and in order to enable the Recipient to evaluate the Transaction, the Recipient hereby commits to keep the Information confidential and to comply with the standstill provisions, all in accordance with this Agreement.

DECLARE TO HAVE AGREED AS FOLLOWS:

## 1. **Definitions**

1.1 In this Agreement the following capitalised words shall have the following meanings:

“**Agreement**” means this Confidentiality and Standstill Agreement;

“**Information**” means any information or data relating to the Transaction, the Group, the Group’s assets and liabilities, the shareholders of the Company and/or any of their respective affiliates, whether received orally, in writing, electronically, visually or in any other form and whether before or after the date of this Agreement, together with all analyses, memoranda, reports, documents, data or information which contain or reflect information or data as aforementioned. “**Information**” does not include any information which:

- (A) was, as at the date of its disclosure to the Recipient, public knowledge, or which subsequently to the date of its disclosure, becomes public knowledge other than by reason of any breach of this Agreement;
- (B) is lawfully obtained by the Recipient through any other source and without any express or implicit obligation of confidentiality.

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- (C) is required to be disclosed by (i) any court of competent jurisdiction or any competent judicial, governmental, supervisory or regulatory body, or (ii) the rules of any stock exchange on which the shares or other securities of the Company are listed; and
- (D) is disclosed with prior written consent of the Company, subject to the conditions set forth in such written consent.

“**Group**” as defined above;

“**Company**” as defined above;

“**Inside Information**” as defined in clause 6.1;

“**Recipient Representative**” as defined in clause 2.6;

“**Parties**” as defined above; and

“**Transaction**” as defined above.

1.2 In this Agreement a reference to the Group, the Recipient or any of their respective affiliates shall include a reference to the managing directors, supervisory directors, shareholders, employees and advisers of the Group, the Recipient and their respective affiliates.

1.3 In this Agreement any reference to a person includes a reference to persons and corporate bodies including partnerships.

1.4 In this Agreement a reference to an “affiliate” shall be construed as a reference to a group company within the meaning of section 2:24b of the Dutch Civil Code.

## 2. **Confidentiality of Information**

2.1 The Recipient shall hold the Information strictly confidential and shall not without the prior written consent of the Company:

- (A) reveal (or cause to be revealed), disclose (or cause to be disclosed), make (or cause to be made) available or publish (or cause to be published) the Information, or any part thereof, in any shape, form or manner; or
- (B) use (or cause to be used) or make (or cause to be made) use of the Information, or any part thereof, for any purpose other than in connection with the Transaction;

it being understood that Information obtained by any Recipient Representative that holds a position with the Company, whether as supervisory board member, advisor, or otherwise, may continue to be used by such person in fulfilling his (statutory) obligations via-à-vis the Company in ordinary course.

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2.2 The Recipient shall keep confidential and not disclose to anyone the existence and/or nature of the negotiations in relation to the Transaction, the existence of this Agreement or the fact that the Information has been made available.

2.3 If the Recipient becomes aware of the confidentiality of Information having been breached or is threatened to be breached, it shall immediately notify the Company of the same and in consultation with the Company take all necessary steps to mitigate the effects of such (threatened) breach.

2.4 The Recipient shall immediately inform (to the extent permitted by law) the Company of any requirement to disclose or request or order for any disclosure referred to in clause 1.1(C) and inform the Company of the full circumstances in relation thereto.

2.5 The restrictions on disclosure and use set out in this Agreement apply similarly to all documents and (computerised) data prepared by or on behalf of the Recipient containing or based on the Information.

2.6 The restrictions on disclosure and use set out in this Agreement apply similarly to the Recipient’s (ultimate) parent compan(y)(ies), its and their affiliates, advisers, agents, representatives, directors and employees (each such party a “**Recipient Representative**”).

- 2.7 The Recipient shall be allowed to make the Information available to a Recipient Representative solely on a need to know basis and provided that these persons are bound by at least equivalent confidentiality obligations and restrictions towards the Recipient. The Recipient shall inform each such Recipient Representative to whom the Information may be made available and to whom Information is disclosed about the restrictions as to the use and the disclosure of the Information set out herein and shall ensure that each such Recipient Representative shall observe those restrictions.
- 2.8 The Recipient is responsible for any breach of this Agreement, which includes a breach by or resulting from a Recipient Representative's breach of any such equivalent confidentiality obligations and restrictions.
3. **Further undertakings by the Recipient**
- 3.1 The Recipient shall not, and shall procure that the Recipient Representatives which were provided with Information shall not, without the prior written consent of the Company, directly or indirectly, contact, solicit or entice away any employee (including directors and managers) of the Company or its subsidiaries or affiliates.
- 3.2 At the earlier event of the termination of this Agreement or the termination of the discussions in respect of the Transaction, the Recipient shall (and shall cause the Recipient Representative's to) return to the Company, at the Company's first request, all Information and all copies thereof. In addition, the Recipient shall (and shall cause the Recipient Representative's to) destroy (or produce evidence of the destruction of) any documents or data (including data held in computerised forms) which contain or are based on the Information.
4. **No representation**
- 4.1 The Recipient acknowledges and agrees that neither the Company, nor any of its officers, employees or advisers (i) make any representation or warranty, express or implied, as to, or assume any responsibility for, the accuracy, reliability or completeness of any of the Information or the assumption on which it is based or (ii) shall be under any obligation to update or correct any inaccuracy in the Information or otherwise be liable to the Recipient or any other person (including any Recipient Representative) in respect to the Information.

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5. **Amendments**
- The terms of this Agreement and the obligations under this Agreement of the Parties may only be amended or modified by written agreement between Parties.
6. **Inside information and Standstill**
- 6.1 The Recipient agrees and acknowledges that some or all of the Information provided, is or may qualify as or contain inside information (*voorwetenschap*) as defined in section 5:53 of the Dutch Act on Financial Supervision (*Wet op het financieel toezicht*) ("**Inside Information**") and that the use of such Inside Information may be regulated or prohibited by applicable legislation relating to insider dealing.
7. **Term**
- 7.1 Unless otherwise provided in this Agreement, and except as otherwise required by applicable laws, this Agreement will remain effective for a period of six months after the date hereof, unless amended or terminated earlier in writing by mutual consent between the Parties hereto.
- 7.2 Subject to clause 7.1, the obligations in this Agreement are continuing and, in particular, shall survive the termination of any discussions or negotiations between the Parties.
8. **General provisions**
- 8.1 The Parties to this Agreement waive their rights, if any, to annul (*vernietigen*), (partly) rescind, (partly) dissolve (*ontbinden*) or cancel this Agreement, or to request annulment, (partly) rescission, (partly) dissolution or cancellation of this Agreement, including on the basis of section 6:228 or 6:265 of the Dutch Civil Code.
- 8.2 This Agreement may be executed in two or more counterparts, each of which shall be deemed an original, but all of which shall constitute one and the same document and that each signatory may deliver a signed copy of this Agreement by fax and that any such faxed copy shall be deemed to be an original for all purposes.
9. **Governing law and Jurisdiction**
- 9.1 This Agreement and any contractual and non contractual obligations arising therefrom shall be governed exclusively by Dutch law.
- 9.2 All disputes arising out of or in connection with this Agreement shall be settled by the competent court in Amsterdam, the Netherlands, subject to appeal and appeal in the second instance. The Parties irrevocably waive any rights that they may have or acquire to object to the jurisdiction of these courts.

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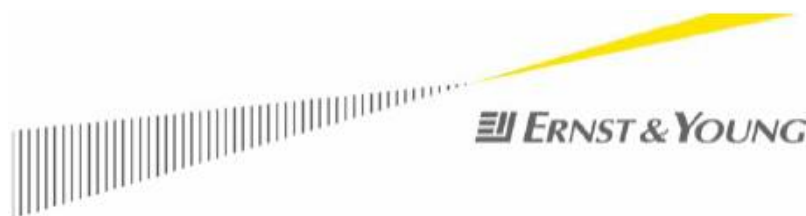
Name:  
Title:  
Date:

Name: L.P.A. Bergstein  
Title: Director  
Date 22/12/11

M.A. van Osch  
Director  
22/12/11

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**SCHEDULE 2 — FAIRNESS OPINION**



**Ernst & Young LLP**  
1 More London Place  
London SE1 2AF

Tel: 020 7951 2000  
Fax: 020 7951 1345  
www.ey.com/uk

17 February 2012

The Special Committee of the Supervisory Board  
Amsterdam Molecular Therapeutics (AMT) Holding N.V.  
Meibergdreef 61  
1105 BA Amsterdam  
The Netherlands

Your ref:  
Our ref: TAS/VBM/ST/SD  
  
Direct line: 020 7951 4302  
Email: staylor11@uk.ey.com

For the attention of F. Verdonck

Dear Sirs

**Amsterdam Molecular Therapeutics (AMT) Holding N.V.**

Related to the proposed transaction (the “Transaction”) to acquire the assets and liabilities of Amsterdam Molecular Therapeutics (AMT) Holding N.V. (“AMT” or the “Company”) in exchange for Depositary Receipts for Ordinary Shares issued by a Foundation Trust (*Stichting Administratiekantoor*) holding Ordinary Shares in the capital of uniQure B.V., the Supervisory Board and Management Board of AMT have requested Ernst & Young Transaction Advisory Services, as independent adviser, to give its opinion as to whether the transaction is fair and reasonable so far as shareholders are concerned. The Transaction would include the Company’s manufacturing facility, portfolio of intellectual property rights and AMT’s employees. The consideration and other terms relating to the Transaction are described in the shareholder circular dated 17 February 2012 (the “Shareholder Circular”).

In arriving at our opinion as set out below, we have, amongst other things:

1. Reviewed certain publicly available business and financial information relating to the Company that we deemed to be relevant;
2. Reviewed certain information, including financial forecasts relating to AMT’s business, the future earnings and cash flow expectations, details of the business assets and liabilities, and prospects for the commercialisation of certain gene therapy related products, as provided to us by the Company;
3. Conducted discussions with members of senior management of the Company concerning the matters described in points 1 and 2 above, as well as the corporate plans, financing strategies and the future financial prospects before and after giving effect to the Transaction;
4. Reviewed a copy of the final draft of the Shareholders Circular dated 17 February 2012;

- 
5. Reviewed the market price and recent historical share price trends for the outstanding shares of the Company and compared them with those of certain publicly traded companies that we deemed to be relevant;
  6. Reviewed the results of operations of the Company and compared them with those of certain publicly traded companies that we deemed to be relevant;
  7. Compared the proposed financial terms of the Transaction with the financial terms of certain other transactions that we deemed to be relevant;
  8. Performed certain valuation analysis related to the potential future cashflows pertaining to the major research and development product assets of the Company;
  9. Held discussions with representatives of the Company’s financial adviser and legal adviser; and
  10. Reviewed such other financial information and taken into account such other matters as we deemed necessary, including our assessment of general economic, market and financial conditions.

In preparing our opinion, we have assumed and relied on the accuracy and completeness of all information supplied or otherwise made available to us, discussed with or reviewed by us, or publicly available. We have not assumed any responsibility for independently verifying such information. In addition, we have not undertaken an independent evaluation or appraisal of any of the assets or liabilities of the Company, or indeed any of the proposed Investors, or been provided with any such evaluation or appraisal nor have we evaluated the solvency or fair value of the Company or any of the Investors themselves under any laws relating to insolvency, bankruptcy, or any similar matters.

Furthermore, we have not assumed any obligation to conduct any physical on-site inspections of AMT’s properties or manufacturing facilities.

In providing this opinion, we have assumed that the Transaction will be completed on the terms described in the draft Shareholders Circular reviewed by us. With respect to the financial forecast information furnished to or discussed with us by the Company, we have assumed that it has been reasonably and diligently prepared and reflects the contemporaneous estimates, judgment and views of the Company's management as to the expected future financial performance of AMT.

We have assumed that in the course of obtaining the necessary regulatory or other consents or approvals (contractual or otherwise) for the Transaction to proceed, no restrictions, including any divestiture requirements or amendments or modifications, will be imposed that will have a material adverse effect on the contemplated benefits of the Transaction.

In connection with the preparation of this opinion, we have not been authorised by the Company to solicit, nor have we solicited, third-party indications of interest for the acquisition of all or any part of the Company.

Our opinion is based upon market, economic, financial and other conditions as they exist, and on the information made available to us, as of the date of this letter.

We will receive a fee from the Company for our services, none of which is contingent upon the completion of the Transaction. We have not provided financial advisory or financing services to the Company, but may do so and may receive fees for the rendering of such services in the future.

This opinion is solely for the use and benefit of the Special Committee of the Supervisory Board of the Company in its evaluation of the Transaction and shall not be used for any other purpose whatsoever. This opinion is not intended to be relied upon or confer any rights or remedies upon

any creditor or employee. This opinion shall not, in whole or in part, be disclosed, disseminated, summarised or referred to, for any purpose other than for the purpose of public disclosure within the final Shareholders Circular related to this Transaction.

Our opinion does not address the merits of the decision by the Company to recommend the Transaction and does not constitute a recommendation to any shareholders as to how they should vote on the proposed Transaction.

On the basis of the above, we are of the opinion that, as of the date of this letter, the Transaction is fair and reasonable so far as AMT shareholders are concerned.

Ernst & Young LLP


Ernst & Young LLP  
United Kingdom

### SCHEDULE 3 — IPR AND FURTHER ASSETS AND LIABILITIES



#### Part 1 — Intellectual Property Rights

“Intellectual Property Rights” means the intellectual property rights of the Group in relation to the Business, such as but not limited to (a) patents, trade marks, service marks, registered designs, trade, business and company names, internet domain names and e-mail addresses, unregistered trade marks and service marks, copyrights, database rights, know how, rights in designs and inventions and applications and rights to apply for any of those rights; (b) the rights to sue for past infringements of any of the foregoing rights, including those set out in the table below.





#### LIST OF TRADEMARKS

	Catchword	Type	Country	Classes	Appl.No.	Appl.date	Reg.No.	Reg.date	Ren.date	Applicant	Status	Case No.	Watch
1.	AMT	Wordmark	BX	01, 05, 42	996846	13-09-01.	696184	13-09-01.	13-09-11.	AMT B.V.	Registered	T17138BX00	Yes
2.	AMT	Wordmark	CA	01, 05, 42	1130879	12-02-02.	630501	19-01-05.	19-01-20.	Amsterdam Molecular Therapeutics (AMT) IP B.V.	Registered	T17138CA00	Yes
3.	AMT	Wordmark	EU	01, 05, 42	2573137	11-02-02.	2573137	03-07-03.	11-02-22.	Amsterdam Molecular Therapeutics (AMT) IP B.V.	Registered	T17138EU00	Yes
4.		Logotype	BX	01, 05, 42	996845	13-09-01.	700080	13-09-01.	13-09-11.	AMT B.V.	Registered	T17139BX00	Yes
5.	AMT	Wordmark	CA	01, 05, 42, 44	1478301	23-04-10.				Amsterdam Molecular Therapeutics (AMT) IP B.V.	Pending	T55829CA00	Yes

6.	AMT	Wordmark	EU	01, 05, 42, 44	8640237	26-10-09.	8640237	10-05-10.	26-10-19.	Amsterdam Molecular Therapeutics (AMT) IP B.V.	Registered	T55829EU00	Yes
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7.	AMT	Wordmark	US	01, 05, 42	85/021857	23-04-10.				Amsterdam Molecular Therapeutics (AMT) IP B.V.	Pending	T55829US00	Yes
8.	AMT	Wordmark	US	44	85/368007	11-07-11.				Amsterdam Molecular Therapeutics (AMT) IP B.V.	Pending	T55829US01	Yes
9.	AMT	Wordmark	CH (WO)	01, 05, 42, 44	8640237-01	23-04-10.	1040425	23-04-10.	23-04-20.	Amsterdam Molecular Therapeutics (AMT) IP B.V.	Registered	T55829WO00	Yes
10.		Logotype	CH	05	536152008	06-04-09.	587323	09-06-09.	06-04-19.	Amsterdam Molecular Therapeutics (AMT) IP B.V.	Registered	T56005CH00	No
11.		Logotype	IL	05	209906	24-03-08.	209906	07-02-10.	24-03-18.	Amsterdam Molecular Therapeutics (AMT) Holding N.V.	Registered	T56005IL00	No

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12.		Logotype	IS	05	10692009	22-04-09.	3772009	02-06-09.	02-06-19.	Amsterdam Molecular Therapeutics (AMT) Holding N.V.	Registered	T56005IS00	No
13.		Logotype	JO	05	100494	23-04-08.	100494	23-04-08.	23-04-18.	Amsterdam Molecular Therapeutics (AMT) Holding N.V.	Registered	T56005JO00	No
14.		Logotype	NO	05	200905089	21-04-09.	251774	14-07-09.	14-07-19.	Amsterdam Molecular Therapeutics (AMT) Holding N.V.	Registered	T56005NO00	No
15.		Logotype	TR	05	200925333	18-05-09.	200925333	04-05-10.	18-05-19.	Amsterdam Molecular Therapeutics (AMT) Holding N.V.	Registered	T56005TR00	No
16.		Logotype	EU	01, 05, 42, 44	8640252	26-10-09.	8640252	10-05-10.	26-10-19.	Amsterdam Molecular Therapeutics (AMT) IP B.V.	Registered	T55830EU00	Yes

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17.		Logotype	US	01, 05, 42, 44	85/021908	23-04-10.				Amsterdam Molecular Therapeutics (AMT) IP B.V.	Pending	T55830US00	Yes
18.		Wordmark	EU	01, 05, 42, 44	9599937	15-12-10.	9599937	26-04-11.	15-12-20.	Amsterdam Molecular Therapeutics (AMT) IP B.V.	Registered	T56839EU00	Indirect watch
19.		Wordmark	US	01, 05, 42, 44	85/338438	06-06-11.				Amsterdam Molecular Therapeutics (AMT) IP B.V.	Pending	T56839US00	Indirect watch
20.		Wordmark	JP (WO)	05	9599937_01	03-06-11.	1089500	03-06-11.	03-06-21.	Amsterdam Molecular Therapeutics (AMT) IP B.V.	Registered	T56839WO00	Indirect watch
21.		Wordmark	TR (WO)	05	9599937_01	03-06-11.	1089500	03-06-11.	03-06-21.	Amsterdam Molecular Therapeutics (AMT) IP B.V.	Registered	T56839WO00	Indirect watch

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22.	DELIVERING CURE	Wordmark	AE	05	113972	03-06-08.	150481	09-12-11.	03-06-18.	Amsterdam Molecular Therapeutics (AMT) Holding N.V.	Registered	T56004AE00	No
23.	DELIVERING CURE	Wordmark	BH	05	64727	18-03-08.				Amsterdam Molecular	Pending	T56004BH00	No

24.	DELIVERING CURE	Wordmark	CA	5	1388257	20-03-08.				Therapeutics (AMT) Holding N.V.	Pending	T56004CA00	No
25.	DELIVERING CURE	Wordmark	IR	05	86122678	18-03-08.	157479	14-09-08.	18-03-18.	Amsterdam Molecular Therapeutics (AMT) IP B.V.	Registered	T56004IR00	No
26.	DELIVERING CURE	Wordmark	JP	05	2008023029	27-03-08.	5343913	06-08-10.	06-08-20.	Amsterdam Molecular Therapeutics (AMT) IP B.V.	Registered	T56004JP00	No
27.	DELIVERING CURE	Wordmark	LB	05	2449	08-04-08.	116062	24-04-08.	24-04-23.	Amsterdam Molecular Therapeutics (AMT) Holding N.V.	Registered	T56004LB00	No
28.	DELIVERING CURE	Wordmark	LY	05	17093	05-02-09.				Amsterdam Molecular Therapeutics (AMT) Holding N.V.	Pending	T56004LY00	No
29.	DELIVERING CURE	Wordmark	MA	05	118083	19-06-08.	118083	17-11-08.	19-06-18.	Amsterdam Molecular Therapeutics (AMT) Holding N.V.	Registered	T56004MA00	No
30.	DELIVERING CURE	Wordmark	OM	05	49398	19-03-08.	49398	11-08-09.	19-03-18.	Amsterdam Molecular Therapeutics (AMT) Holding N.V.	Registered	T56004OM00	No

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31.	DELIVERING CURE	Wordmark	QA	05	50165	03-04-08.	50165	20-03-11.	03-04-18.	Amsterdam Molecular Therapeutics (AMT) Holding N.V.	Registered	T56004QA00	No
32.	DELIVERING CURE	Wordmark	RU	05	2008707490	14-03-08.	381651	16-06-09.	14-03-18.	Amsterdam Molecular Therapeutics (AMT) Holding N.V.	Registered	T56004RU00	No
33.	DELIVERING CURE	Wordmark	SY	05	3814	22-04-08.				Amsterdam Molecular Therapeutics (AMT) Holding N.V.	Pending	T56004SY00	No
34.	DELIVERING CURE	Wordmark	TN	05	EE080755	19-03-08.	EE080755	26-01-10.	19-03-18.	Amsterdam Molecular Therapeutics (AMT) Holding N.V.	Registered	T56004TN00	No
35.	DELIVERING CURE	Wordmark	US	05	77/421590	13-03-08.				Amsterdam Molecular Therapeutics (AMT) Holding N.V.	Pending	T56004US00	No
36.	DELIVERING CURE	Wordmark	ZA	05	200805836	14-03-08.	200805836	14-03-08.	14-03-18.	Amsterdam Molecular Therapeutics (AMT) Holding N.V.	Registered	T56004ZA00	No
37.		Logotype	EU	05, 44	8640609	26-10-09.	8640609	10-05-10.	26-10-19.	Amsterdam Molecular Therapeutics (AMT) IP B.V.	Registered	T55831EU00	Yes
38.		Logotype	US	05, 44	85/021938	23-04-10.				Amsterdam Molecular Therapeutics (AMT) IP B.V.	Pending	T55831US00	Yes

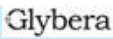
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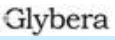
39.	GLYBERA	Wordmark	AE	05	101941	31-10-07.				Amsterdam Molecular Therapeutics (AMT) Holding N.V.	Pending	T56001AE00	Yes
40.	GLYBERA	Wordmark	AU	05	1176048	14-05-07.	1176048	12-12-07.	14-05-17.	Amsterdam Molecular Therapeutics (AMT) Holding N.V.	Registered	T56001AU00	Yes

41.	GLYBERA	Wordmark	BH	05	62689	07-01-08.	62689	07-01-08.	07-01-18.	Amsterdam Molecular Therapeutics (AMT) Holding N.V.	Registered	T56001BH00	Yes
42.	GLYBERA	Wordmark	CA	5	1355754	16-07-07.				Amsterdam Molecular Therapeutics (AMT) IP B.V.	Pending	T56001CA00	Yes
43.	GLYBERA	Wordmark	CH	05	551392007	14-05-07.	562178	11-09-07.	14-05-17.	Amsterdam Molecular Therapeutics (AMT) IP B.V.	Registered	T56001CH00	Yes
44.	GLYBERA	Wordmark	DZ	05	72791	24-10-07.				Amsterdam Molecular Therapeutics (AMT) Holding N.V.	Pending	T56001DZ00	Yes
45.	GLYBERA	Wordmark	EG	05	208229	22-10-07.				Amsterdam Molecular Therapeutics (AMT) Holding N.V.	Pending	T56001EG00	Yes
46.	GLYBERA	Wordmark	EU	05, 44	5901269	01-05-07.	5901269	14-05-09.	01-05-17.	Amsterdam Molecular Therapeutics (AMT) IP B.V.	Registered	T56001EU00	Yes
47.	GLYBERA	Wordmark	IL	05	204800	21-10-07.	204800	11-08-09.	21-10-17.	Amsterdam Molecular Therapeutics (AMT) Holding N.V.	Registered	T56001IL00	Yes

48.	GLYBERA	Wordmark	IS	05	14642007	14-05-07.	8122007	04-07-07.	04-07-17.	Amsterdam Molecular Therapeutics (AMT) Holding N.V.	Registered	T56001IS00	Yes
49.	GLYBERA	Wordmark	JO	05	99133	24-10-07.	99133	01-05-07.	01-05-17.	Amsterdam Molecular Therapeutics (AMT) Holding N.V.	Registered	T56001JO00	Yes
50.	GLYBERA	Wordmark	JP	05	2007054257	30-05-07.	5088657	02-11-07.	02-11-17.	Amsterdam Molecular Therapeutics (AMT) IP B.V.	Registered	T56001JP00	Yes
51.	GLYBERA	Wordmark	LB	05	6612	23-10-07.	113370	25-10-07.	25-10-22.	Amsterdam Molecular Therapeutics (AMT) Holding N.V.	Registered	T56001LB00	Yes
52.	GLYBERA	Wordmark	LY	05	16593	22-12-08.				Amsterdam Molecular Therapeutics (AMT) Holding N.V.	Pending	T56001LY00	Yes
53.	GLYBERA	Wordmark	MA	05	113550	23-10-07.	113550	23-10-07.	23-10-17.	Amsterdam Molecular Therapeutics (AMT) Holding N.V.	Registered	T56001MA00	Yes
54.	GLYBERA	Wordmark	NO	05	200705606	15-05-07.	241553	19-10-07.	19-10-17.	Amsterdam Molecular Therapeutics (AMT) Holding N.V.	Registered	T56001NO00	Yes
55.	GLYBERA	Wordmark	NZ	05	768310	14-05-07.	768310	15-11-07.	14-05-17.	Amsterdam Molecular Therapeutics (AMT) Holding N.V.	Registered	T56001NZ00	Yes
56.	GLYBERA	Wordmark	OM	05	47462	22-10-07.	47462	24-08-08.	22-10-17.	Amsterdam Molecular Therapeutics (AMT) Holding N.V.	Registered	T56001OM00	Yes

57.	GLYBERA	Wordmark	QA	05	47253	31-10-07.	47253	31-12-09.	31-10-17.	Amsterdam Molecular Therapeutics (AMT) Holding N.V.	Registered	T56001QA00	Yes
58.	GLYBERA	Wordmark	RU	05	2008707340	13-03-08.	377215	20-04-09.	13-03-18.	Amsterdam Molecular	Registered	T56001RU00	Yes

										Therapeutics (AMT) Holding N.V.			
59.	GLYBERA	Wordmark	SA	05	125692	12-01-08.	1156/45	25-04-10.	12-09-17.	Amsterdam Molecular Therapeutics (AMT) Holding N.V.	Registered	T56001SA00	Yes
60.	GLYBERA	Wordmark	SY	05	4268	28-10-07.				Amsterdam Molecular Therapeutics (AMT) Holding N.V.	Pending	T56001SY00	Yes
61.	GLYBERA	Wordmark	TN	05	EE072667	24-10-07.	EE072667	19-05-09.	24-10-17.	Amsterdam Molecular Therapeutics (AMT) Holding N.V.	Registered	T56001TN00	Yes
62.	GLYBERA	Wordmark	TR	05	2007026778	17-05-07.	200726778	17-05-07.	17-05-17.	Amsterdam Molecular Therapeutics (AMT) Holding N.V.	Registered	T56001TR00	Yes
63.	GLYBERA	Wordmark	US	05	77/179356	11-05-07.	3972244	07-06-11.	07-06-21.	Amsterdam Molecular Therapeutics (AMT) IP B.V.	Registered	T56001US00	Yes
64.	GLYBERA	Wordmark	ZA	05	200723919	19-10-07.	2007/23919	19-10-07.	19-10-17.	Amsterdam Molecular Therapeutics (AMT) Holding N.V.	Registered	T56001ZA00	Yes
65.		Logotype	EU	05, 44	8640641	26-10-09.	8640641	10-05-10.	26-10-19.	Amsterdam Molecular Therapeutics (AMT) IP B.V.	Registered	T55832EU00	Indirect watch

66.		Logotype	US	05, 44	85/021985	23-04-10.				Amsterdam Molecular Therapeutics (AMT) IP B.V.	Pending	T55832US00	Indirect watch
67.	LPLCHIP	Wordmark	CA	1, 10, 42, 44, 5, 9	1474070	22-03-10.				Amsterdam Molecular Therapeutics (AMT) IP B.V.	Pending	T55770CA00	Yes
68.	LPLCHIP	Wordmark	EU	01, 05, 09, 10, 42, 44	8590911	02-10-09.	8590911	31-05-10.	02-10-19.	Amsterdam Molecular Therapeutics (AMT) IP B.V.	Registered	T55770EU00	Yes
69.	LPLCHIP	Wordmark	US	1, 5, 9	77/964892	22-03-10.	3972502	07-06-11.	07-06-21.	Amsterdam Molecular Therapeutics (AMT) IP B.V.	Registered	T55770US00	Yes
70.	LPLCHIP	Wordmark	CH (WO)	01, 05, 09, 10	8590911-01	06-04-10.	1036745	06-04-10.	06-04-20.	Amsterdam Molecular Therapeutics (AMT) IP B.V.	Registered	T55770WO00	Yes
71.	LPLCHIP	Wordmark	IS (WO)	01, 05, 09, 10	8590911-01	06-04-10.	1036745	06-04-10.	06-04-20.	Amsterdam Molecular Therapeutics (AMT) IP B.V.	Registered	T55770WO00	Yes
72.	LPLCHIP	Wordmark	NO (WO)	01, 05, 09, 10	8590911-01	06-04-10.	1036745	06-04-10.	06-04-20.	Amsterdam Molecular Therapeutics (AMT) IP B.V.	Registered	T55770WO00	Yes
73.	VECTIPRO	Wordmark	AE	05	101942	31-10-07.	100927	24-03-10.	31-10-17.	Amsterdam Molecular Therapeutics (AMT) Holding N.V.	Registered	T56002AE00	Yes
74.	VECTIPRO	Wordmark	AU	05	1176051	14-05-07.	1176051	12-12-07.	14-05-17.	Amsterdam Molecular Therapeutics (AMT) Holding N.V.	Registered	T56002AU00	Yes

75.	VECTIPRO	Wordmark	BH	05	62690	07-01-08.	62690	07-01-08.	07-01-18.	Amsterdam Molecular Therapeutics (AMT) Holding N.V.	Registered	T56002BH00	Yes
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76.	VECTIPRO	Wordmark	CA	5	1355761	16-07-07.				Amsterdam Molecular Therapeutics (AMT) IP B.V.	Pending	T56002CA00	Yes
77.	VECTIPRO	Wordmark	CH	05	551382007	14-05-07.	562177	11-09-07.	14-05-17.	Amsterdam Molecular Therapeutics (AMT) IP B.V.	Registered	T56002CH00	Yes
78.	VECTIPRO	Wordmark	DZ	05	72793	24-10-07.				Amsterdam Molecular Therapeutics (AMT) Holding N.V.	Pending	T56002DZ00	Yes
79.	VECTIPRO	Wordmark	EG	05	208203	22-10-07.				Amsterdam Molecular Therapeutics (AMT) Holding N.V.	Pending	T56002EG00	Yes
80.	VECTIPRO	Wordmark	EU	05	5901277	01-05-07.	5901277	10-04-08.	01-05-17.	Amsterdam Molecular Therapeutics (AMT) IP B.V.	Registered	T56002EU00	Yes
81.	VECTIPRO	Wordmark	IL	05	204915	23-10-07.	204915	11-08-09.	23-10-17.	Amsterdam Molecular Therapeutics (AMT) Holding N.V.	Registered	T56002IL00	Yes
82.	VECTIPRO	Wordmark	IR	05	86091403	08-12-07.	157475	14-09-08.	08-12-17.	Amsterdam Molecular Therapeutics (AMT) Holding N.V.	Registered	T56002IR00	Yes
83.	VECTIPRO	Wordmark	IS	05	14632007	14-05-07.	8112007	04-07-07.	04-07-17.	Amsterdam Molecular Therapeutics (AMT) Holding N.V.	Registered	T56002IS00	Yes

84.	VECTIPRO	Wordmark	JO	05	99366	24-10-07.	99366	14-01-09.	01-05-17.	Amsterdam Molecular Therapeutics (AMT) Holding N.V.	Registered	T56002JO00	Yes
85.	VECTIPRO	Wordmark	JP	05	2007054258	30-05-07.	5088658	02-11-07.	02-11-17.	Amsterdam Molecular Therapeutics (AMT) IP B.V.	Registered	T56002JP00	Yes
86.	VECTIPRO	Wordmark	LB	05	6622	23-10-07.	113434	30-10-07.	30-10-22.	Amsterdam Molecular Therapeutics (AMT) Holding N.V.	Registered	T56002LB00	Yes
87.	VECTIPRO	Wordmark	LY	05	16595	22-12-08.				Amsterdam Molecular Therapeutics (AMT) Holding N.V.	Pending	T56002LY00	Yes
88.	VECTIPRO	Wordmark	MA	05	113551	23-10-07.	113551	23-10-07.	23-10-17.	Amsterdam Molecular Therapeutics (AMT) Holding N.V.	Registered	T56002MA00	Yes
89.	VECTIPRO	Wordmark	NO	05	200705604	15-05-07.	241558	22-10-07.	22-10-17.	Amsterdam Molecular Therapeutics (AMT) Holding N.V.	Registered	T56002NO00	Yes
90.	VECTIPRO	Wordmark	NZ	05	768309	14-05-07.	768309	12-02-09.	01-05-17.	Amsterdam Molecular Therapeutics (AMT) Holding N.V.	Registered	T56002NZ00	Yes
91.	VECTIPRO	Wordmark	OM	05	47461	22-10-07.	47461	30-05-09.	22-10-17.	Amsterdam Molecular Therapeutics (AMT) Holding N.V.	Registered	T56002OM00	Yes
92.	VECTIPRO	Wordmark	QA	05	47255	31-10-07.	47255	31-12-09.	31-10-17.	Amsterdam Molecular Therapeutics (AMT) Holding N.V.	Pending	T56002QA00	Yes

93.	VECTIPRO	Wordmark	RU	05	2008707342	13-03-08.	381400	10-06-09.	13-03-18.	Amsterdam Molecular	Registered	T56002RU00	Yes
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										Therapeutics (AMT) Holding N.V.			
94.	VECTIPRO	Wordmark	SA	05	125693	12-01-08.	1171/48	19-06-10.	12-09-17.	Amsterdam Molecular Therapeutics (AMT) Holding N.V.	Registered	T56002SA00	Yes
95.	VECTIPRO	Wordmark	SY	05	4269	28-10-07.				Amsterdam Molecular Therapeutics (AMT) Holding N.V.	Pending	T56002SY00	Yes
96.	VECTIPRO	Wordmark	TN	05	EE072666	24-10-07.	EE072666	19-05-09.	24-10-17.	Amsterdam Molecular Therapeutics (AMT) Holding N.V.	Registered	T56002TN00	Yes
97.	VECTIPRO	Wordmark	TR	05	2007026779	17-05-07.	200726779	17-05-07.	17-05-17.	Amsterdam Molecular Therapeutics (AMT) Holding N.V.	Registered	T56002TR00	Yes
98.	VECTIPRO	Wordmark	US	05	77/179357	11-05-07.	3703954	03-11-09.	03-11-19.	Amsterdam Molecular Therapeutics (AMT) IP B.V.	Registered	T56002US00	Yes
99.	VECTIPRO	Wordmark	ZA	05	200723918	19-10-07.	2007/23918	19-10-07.	19-10-17.	Amsterdam Molecular Therapeutics (AMT) Holding N.V.	Registered	T56002ZA00	Yes
100.	ZYAMTIN	Wordmark	AE	05	101943	31-10-07.	100909	24-03-10.	31-10-17.	Amsterdam Molecular Therapeutics (AMT) Holding N.V.	Registered	T56003AE00	Yes
101.	ZYAMTIN	Wordmark	AU	05	1176049	14-05-07.	1176049	12-12-07.	14-05-17.	Amsterdam Molecular Therapeutics (AMT) Holding N.V.	Registered	T56003AU00	Yes

102.	ZYAMTIN	Wordmark	BH	05	62691	07-01-08.	62691	07-01-08.	07-01-18.	Amsterdam Molecular Therapeutics (AMT) Holding N.V.	Registered	T56003BH00	Yes
103.	ZYAMTIN	Wordmark	CA	5	1355762	16-07-07.				Amsterdam Molecular Therapeutics (AMT) IP B.V.	Pending	T56003CA00	Yes
104.	ZYAMTIN	Wordmark	CH	05	551982007	15-05-07.	562360	13-09-07.	15-05-17.	Amsterdam Molecular Therapeutics (AMT) IP B.V.	Registered	T56003CH00	Yes
105.	ZYAMTIN	Wordmark	DZ	05	72792	24-10-07.				Amsterdam Molecular Therapeutics (AMT) Holding N.V.	Pending	T56003DZ00	Yes
106.	ZYAMTIN	Wordmark	EG	05	208231	22-10-07.				Amsterdam Molecular Therapeutics (AMT) Holding N.V.	Pending	T56003EG00	Yes
107.	ZYAMTIN	Wordmark	EU	05, 44	5901251	01-05-07.	5901251	22-01-09.	01-05-17.	Amsterdam Molecular Therapeutics (AMT) IP B.V.	Registered	T56003EU00	Yes
108.	ZYAMTIN	Wordmark	IL	05	204799	21-10-07.	204799	11-04-09.	21-10-17.	Amsterdam Molecular Therapeutics (AMT) Holding N.V.	Registered	T56003IL00	Yes
109.	ZYAMTIN	Wordmark	IR	05	86091401	08-12-07.	158201	14-09-08.	08-12-17.	Amsterdam Molecular Therapeutics (AMT) Holding N.V.	Registered	T56003IR00	Yes
110.	ZYAMTIN	Wordmark	IS	05	14652007	14-05-07.	8132007	04-07-07.	04-07-17.	Amsterdam Molecular Therapeutics (AMT) Holding N.V.	Registered	T56003IS00	Yes



111.	ZYAMTIN	Wordmark	JO	05	99208	24-10-07.	99208	03-03-09.	01-05-17.	Amsterdam Molecular Therapeutics (AMT) Holding N.V.	Registered	T56003JO00	Yes
112.	ZYAMTIN	Wordmark	JP	05	2007054259	30-05-07.	5088659	02-11-07.	02-11-17.	Amsterdam Molecular Therapeutics (AMT) IP B.V.	Registered	T56003JP00	Yes
113.	ZYAMTIN	Wordmark	LB	05	6623	23-10-07.	113437	30-10-07.	30-10-22.	Amsterdam Molecular Therapeutics (AMT) Holding N.V.	Registered	T56003LB00	Yes
114.	ZYAMTIN	Wordmark	LY	05	16594	22-12-08.				Amsterdam Molecular Therapeutics (AMT) Holding N.V.	Pending	T56003LY00	Yes
115.	ZYAMTIN	Wordmark	MA	05	113552	23-10-07.	113552	23-10-07.	23-10-17.	Amsterdam Molecular Therapeutics (AMT) Holding N.V.	Registered	T56003MA00	Yes
116.	ZYAMTIN	Wordmark	NO	05	200705605	15-05-07.	241517	18-10-07.	18-10-17.	Amsterdam Molecular Therapeutics (AMT) Holding N.V.	Registered	T56003NO00	Yes
117.	ZYAMTIN	Wordmark	NZ	05	768311	14-05-07.	768311	15-11-07.	14-05-17.	Amsterdam Molecular Therapeutics (AMT) Holding N.V.	Registered	T56003NZ00	Yes
118.	ZYAMTIN	Wordmark	OM	05	47463	22-10-07.	47463	30-05-09.	22-10-17.	Amsterdam Molecular Therapeutics (AMT) Holding N.V.	Registered	T56003OM00	Yes
119.	ZYAMTIN	Wordmark	QA	05	47254	31-10-07.	47254	31-12-09.	31-10-17.	Amsterdam Molecular Therapeutics (AMT) Holding N.V.	Registered	T56003QA00	Yes

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120.	ZYAMTIN	Wordmark	RU	05	2008707341	13-03-08.	394999	01-12-09.	13-03-18.	Amsterdam Molecular Therapeutics (AMT) Holding N.V.	Registered	T56003RU00	Yes
121.	ZYAMTIN	Wordmark	SA	05	125694	12-01-08.	1171/49	19-06-10.	12-09-17.	Amsterdam Molecular Therapeutics (AMT) Holding N.V.	Registered	T56003SA00	Yes
122.	ZYAMTIN	Wordmark	SY	05	4267	28-10-07.				Amsterdam Molecular Therapeutics (AMT) Holding N.V.	Pending	T56003SY00	Yes
123.	ZYAMTIN	Wordmark	TN	05	EE072668	24-10-07.	EE072668	19-05-09.	24-10-17.	Amsterdam Molecular Therapeutics (AMT) Holding N.V.	Registered	T56003TN00	Yes
124.	ZYAMTIN	Wordmark	TR	05	2007026780	17-05-07.	200726780	07-04-08.	17-05-17.	Amsterdam Molecular Therapeutics (AMT) Holding N.V.	Registered	T56003TR00	Yes
125.	ZYAMTIN	Wordmark	US	05	77/179359	11-05-07.	3855311	05-10-10.	05-10-20.		Registered	T56003US00	Yes
126.	ZYAMTIN	Wordmark	ZA	05	200723917	19-10-07.	200723917	14-07-10.	19-10-17.	Amsterdam Molecular Therapeutics (AMT) Holding N.V.	Registered	T56003ZA00	Yes

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## LIST OF PATENTS

No.	Description patent	Owned or Co-Owned by	AMT reference No	Date of filing provisional application	Patent number	[Other]
<b>Strategic Partnerships/Licenses</b>						
	Lipoprotein lipase (LPL) variant therapeutics	Amsterdam Molecular Therapeutics B.V.	AMT-P101	24 June 1999	PCT/CA00/00762	
	IL-10 gene transfer to peripheral	Amsterdam Molecular	AMT-P102	07 March 2002	PCT/NL03/00170	

mononuclear cells	Therapeutics B.V.			
Treatment of non-alcoholic steatotic hepatitis (NASH)	Amsterdam Molecular Therapeutics B.V.	AMT-P103	20 June 2005	PCT/NL05/000446
Improved AAV vectors produced in insect cells	Amsterdam Molecular Therapeutics B.V.	AMT-P104	20 October 2005	PCT/NL06/050262
Vectors with modified initiation codons for the translation of AAV-Rep78 useful for the production of AAV in insect cells	Amsterdam Molecular Therapeutics B.V.	AMT-P105	21 June 2006	PCT/NL07/050298
Use of replication machinery for improved protein production	Amsterdam Molecular Therapeutics B.V.	AMT-P106	19 September 2007	PCT/NL08/050613
Baculovirus vectors comprising repeated coding sequences with different codon biases	Amsterdam Molecular Therapeutics B.V.	AMT-P107	26 July 2007	PCT/NL08/050512

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## LIST OF PATENTS

No.	Description patent	Owned or Co-Owned by	AMT reference No	Date of filing provisional application	Patent number	[Other]
	Optimization of expression of parvoviral rep and cap proteins in insect cells	Amsterdam Molecular Therapeutics (AMT) IP B.V.	AMT-P108	19 February 2008	PCT/NL90/050076	
	Parvoviral capsids with incorporated Gly-Ala repeat region	Amsterdam Molecular Therapeutics (AMT) IP B.V.	AMT-P109	17 June 2008	PCT/NL09/050352	
	Porphobilinogen deaminase gene therapy	Amsterdam Molecular Therapeutics (AMT) IP B.V.	AMT-P110	29 September 2008	PCT/NL09/050584	
	Alanine-Glyoxylate aminotransferase therapeutics	Amsterdam Molecular Therapeutics (AMT) IP B.V.	AMT-P111	30 January 2009	PCT/NL10/050044	
	Use of lipoprotein lipase (LPL) in therapy	Amsterdam Molecular Therapeutics (AMT) IP B.V.	AMT-P112	18 May 2009	PCT/NL10/050294	
	Removal of contaminating viruses from AAV preparations	Amsterdam Molecular Therapeutics (AMT) IP B.V.	AMT-P113	08 September 2011	11180594.1	
	Modified snRNA for use in therapy	Amsterdam Molecular Therapeutics (AMT) IP B.V.	AMT-P116	08 June 2011	61/532,176 PCT/IB11/050584	

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## LIST OF PATENTS

No.	Description patent	Owned or Co-Owned by	AMT reference No	Date of filing provisional application	Patent number	[Other]
	Mutated Rep encoding sequences for use in AAV production	Amsterdam Molecular Therapeutics (AMT) IP B.V.	AMT-P117	11 March 2010	PCT/NL11/050170	
	Method for identifying variant Rep protein encoding nucleic acids	Amsterdam Molecular Therapeutics (AMT) IP B.V.	AMT-P118	11 March 2010	PCT/NL11/050171	
	Monomeric duplex AAV vectors	Amsterdam Molecular Therapeutics (AMT) IP B.V.	AMT-P119	01 April 2011	PCT/NL11/050221	
	Method for determining efficacy of therapy and determining presence or risk of disease	Amsterdam Molecular Therapeutics (AMT) IP B.V.	AMT-P120	02 June 2010	PCT? NL11/050399	
	Method for the preparation of cells	Amsterdam Molecular Therapeutics (AMT) IP B.V.	AMT-P121	31 March 2011	11160727.1	
					61/470,033	

## Part 2 - Contracts

“**Contracts**” means all contracts entered into by the Group relating to the Business and all (existing and future) rights and obligations thereunder, including those set out in the table below, but excluding the Excluded Contracts.

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## LIST OF CONTRACTS

No.	Description agreement	Date	Parties	Change of Control as result of Kairos	Remarks	Outstanding items
<b>Strategic Partnerships/Licenses</b>						
1.	Collaboration with CIMA					
a	license agreement	21-05-2010	AMT B.V.	No	Certain provisions of old agreement are or may still be in force.	The 2005 Agreements, the 2007 Agreements, the Privileged Access Agreement the Virus encode IGF License should be checked for CoC.

b	collaborative development agreement	21-05-2010	AMT B.V.	No	Certain provisions of old agreement are or may still be in force.	The 2005 Agreements, the 2007 Agreements, the Privileged Access Agreement the Virus encode IGF License should be checked for CoC.
2.	Collaboration with ST. Jude:		AMT B.V.			
a	sponsored research agreement	07-07-2007	AMT B.V.	No	Laws of the State of New York apply	
b	license agreement	07-07-2007	AMT B.V.	No	Laws of the State of New York apply	
c	AMT technology agreement	07-07-2007	AMT B.V.	No	Laws of the State of New York apply	

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## LIST OF CONTRACTS

No.	Description agreement	Date	Parties	Change of Control as result of Kairos	Remarks	Outstanding items
d	research license agreement	27-10-2009	AMT B.V.	No	Laws of the State of Tennessee apply	
3.	Collaboration Children's Hospital of Philadelphia					
a	Research collaboration agreement	[08-03-2007]	AMT B.V.	No	Laws of Commonwealth of Pennsylvania apply	
b	License agreement	[29-03-2010]	AMT B.V.	No	Laws of Commonwealth of Pennsylvania apply	
4.	La Sapienza – License and Sponsored Research Agreement	[09]-05-2009	AMT B.V.	No	Laws of England and Wales apply	
5.	Non-exclusive worldwide commercial sub-license agreement with AskBio [in relation to the intra-muscular administration of Glybera® for LPLD].	03-09-2010	AMT B.V.	No	Laws of the State of New York apply	
6.	Non-exclusive worldwide commercial license agreement with Protein Sciences Corporation in relation to the use of <i>Spodoptera Frugiperda</i> cells in relation to the AAV vector used in the manufacture of Glybera® for LPLD.	22-03-2007	AMT B.V.	No	Laws of the State of Connecticut	[DOUBLE CHECK]

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## LIST OF CONTRACTS

No.	Description agreement	Date	Parties	Change of Control as result of Kairos	Remarks	Outstanding items
7.	An exclusive worldwide commercial (sub)license agreement with Xenon Genetics Inc., under which AMT has obtained worldwide rights to use, manufacture and commercialize intellectual property covering certain LPL	18-06-2001	OLD AMT B.V.	No	Laws of the State of California	

	genes in the field of gene therapy to treat LPL deficiency and coronary artery disease.				
8.	Non-exclusive worldwide commercial license agreement with Salk Institute for Biological Studies, under which AMT has obtained rights to commercialize technology that is a component for Glybera for LPLD.	08-02-2008	AMT B.V.	No	Laws of the State of California apply
9.	An exclusive worldwide commercial license agreement with a Sanofi Aventis on, under which AMT has obtained rights in the major markets to use, develop, manufacture and commercialize intellectual	20-12-2006	OLD AMT B.V.	No	Laws of France apply

## LIST OF CONTRACTS

No.	Description agreement	Date	Parties	Change of Control as result of Kairos	Remarks	Outstanding items
	property covering a LPL gene in the field of gene therapy to treat LPL deficiency. This agreement required AMT to pay an upfront signature fee and requires AMT to pay both milestones and royalties to the licensor.					
10.	Non-exclusive worldwide commercial license agreement with the National Institutes of Health re – amongst others - production of AAV in insect cells and AAV 5 Vector.	02-05-2007 (as amended)	AMT B.V.	No	Laws of District of Colombia apply	
11.	Development and commercialisation agreement Progenika	07-08-2009	AMT B.V.	No	Laws of England apply	
12.	Non-exclusive worldwide commercial sublicense agreement with Targeted Genetics Corporation, under which AMT has obtained worldwide rights to commercialize the AAV1 capsid serotype used in Glybera® for LPLD.	05-12- 2006	AMT B.V.	No	Laws of Commonwealth of Pennsylvania apply	

## LIST OF CONTRACTS

No.	Description agreement	Date	Parties	Change of Control as result of Kairos	Remarks	Outstanding items
13.	License agreement with AMGEN re GDNF	30-11-2010	AMT B.V.	[No]	Laws of the State of New York apply	We have not been provided with the executed version.

14.	Development and manufacturing agreement with Institut Pasteur re Sanfilippo	[07-01-2011]				Complete documentation to be submitted
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## Grants

15.	Senternovem innovatiekrediet	18-12-2009	AMT N.V.	<p>In the event of a transfer of knowhow or results originating from the project, the Minister must be informed and he may decide that the credit must be repaid immediately.</p> <p>NB: Section 3.10 of the Subsidy Scheme Innovate (<i>Subsidieregeling innoveren</i>) states: that in the event the shares in the grantee (AMT) are being sold, the Minister may decide that (i)</p>	The Senternovem innovatiekrediet must be transferred as it is in the name of AMT N.V.	AMT N.V. had pledged the assets in relation to the Project (Duchenne).
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## LIST OF CONTRACTS

No.	Description agreement	Date	Parties	Change of Control as result of Kairos	Remarks	Outstanding items
				the grant must be repaid at once or (i) the repayment is being accelerated.		
16.	Senter - TOK 000106 – LPL deficiency	27-06-2001	OLD AMT B.V.	AMT has committed not to transfer IP resulting from the project or assets financed by the credit. Check clause 5 re Demerger.		
17.	Senter – TOP – Il vivo gentherapy	12-03-2002	OLD AMT B.V.	AMT has committed not to transfer IP resulting from the project or assets financed by the credit. Check clause 5 re Demerger.		OLD AMT B.V. has pledged the assets (Duchenne) in relation to the this credit.
18.	Seventh framework programme – Treatrush (no. 242013)	01-02-2010	AMT [unclear which entity].  Agreements is signed by P. Morgan as CFO.	Section II.38 under (h) of Annex II to the grant agreement states that the Commission may terminate the grant agreement where a legal, financial,	<p>The Agreement needs to be transferred if it is in the name of AMT N.V.</p> <p>The change of control clause <u>could</u> be triggered in the event AMT B.V. is contract party</p>	<p>It is unclear which AMT entity is party to the agreement. Assuming AMT N.V. is the party to the agreement the agreement will need to be transferred.</p> <p>Furthermore the grant</p>

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## LIST OF CONTRACTS

No.	Description agreement	Date	Parties	Change of Control as result of Kairos	Remarks	Outstanding items
				organisational or technical change or change of control of a beneficiary (AMT [N.V./B.V.]) calls into question the decision of the Commission to accept its participation.		agreement needs to be reviewed.
19.	Proposal for grant agreement – AIP Digna	[05]-07-2010	AMT B.V.		The documents we reviewed appear to be the	Complete documentation to be submitted.

grant (no. 261506)	proposal for a grant (under the Seventh framework programme). The proposal does not contain a change of control. However, depending on the applicable provisions (e.g. dee 18 above) a change of control may be applicable.
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Dilutive Instruments

20.	Convertible loan agreement Forbion	22-12-2009	AMT N.V.	No	The Agreement needs to be transferred as it is in the name of AMT N.V.
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Lease Agreements

LIST OF CONTRACTS

No.	Description agreement	Date	Parties	Change of Control as result of Kairos	Remarks	Outstanding items
21.	(Sub)Lease agreements with AMC and BDDA with OncoMethylome Sciences, AMT and AVP relating to:	various	OLD AMT B.V.	No	<p>Pursuant to annual accounts only the following leases exist:</p> <p>Agreement between BDDA and AMT regarding leasehold improvements “Meibergdreef 61” as from October 2005 for 11 years. The rent of the leasehold improvements amounts to € 30,000 per year. The lease contract contains an option to extend the lease for another 5 years. The company has the right to cancel the lease earlier on a one-year term however, the company will then need to repay the remaining amount of leased leasehold improvements.</p> <p>– Agreement between BDDA and AMT regarding leasehold improvements “Meibergdreef 57” as from July 2006 for 10 years and 3 months. The rent of the leasehold improvements amounts to € 23,000 per year. The lease</p>	

LIST OF CONTRACTS

No.	Description agreement	Date	Parties	Change of Control as result of Kairos	Remarks	Outstanding items
					contract contains an option to extend the lease for another 5 years.	
a	983 m2 on the second floor ( <i>1e etage</i> ) of Meibergdreef 57	[11]-01-2007	Old AMT B.V.	No		
c	968 m2 on the third floor ( <i>tweede etage</i> ) between OncoMethylome Sciences B.V and BDDA	31-12-2006		No		
		[15-02-2007]	Old AMT B.V			

	and a sublease agreement re a third of the 968 m2 on the third floor ( <i>tweede etage</i> ) between OncoMethylome Sciences B.V and AMT B.V.			
b	an amendment to the sublease agreement with OncoMethylome Sciences	[09-05-2007	Old AMT B.V	No
e	(termination of) old (first and second) rental agreements	16-06-2007	Old AMT B.V	No
f	605 m2 on the fourth floor ( <i>derde etage</i> ) (Meibergdreef 61).	13-10-2007	OLD AMT B.V.	No

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## LIST OF CONTRACTS

No.	Description agreement	Date	Parties	Change of Control as result of Kairos	Remarks	Outstanding items
g	256m2 on the fifth floor ( <i>vierde etage</i> ) between AVP and BDDA	01-10-2005	AVP	No		
h	A GMP facility on Meibergdreef 61 between AVP and BDDA	01-07-2001	AVP	No		

## Pension / Insurance

22.	Pension	[-]-07-2008 / 07-04-2010	AMT B.V.	No		
23.	D&O Insurance	[06-06-2010]	AMT N.V.			The most up to date schedule to the insurance agreement to be reviewed.

## Other

24.	Named patients supply agreement with Pioneer Market Acces Consulting (as amended)	05-05-2010	AMT B.V.	No		
25.	Employment agreement Mr. Jörn Aldag	[·]-10-2009	AMT N.V.	Pursuant to section 1.4 of the Agreement, in the event of (a) new controlling shareholder(s) of AMT N.V. decide not to maintain Mr. Aldag as CEO (or a position		

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## LIST OF CONTRACTS

No.	Description agreement	Date	Parties	Change of Control as result of Kairos	Remarks	Outstanding items
					at least equivalent) then Mr. Aldag has the right to terminate the agreement within two months.	

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1. The Purchaser is duly organised and validly existing as a *besloten vennootschap met beperkte aansprakelijkheid* under the laws of the Netherlands and the Purchaser has all requisite power to enter into the Agreement.
2. The Purchaser is duly registered with the trade register held by the competent Chamber of Commerce.
3. The Purchaser has not been dissolved and no resolution to dissolve the Purchaser has been adopted.
4. The Purchaser has not requested nor been granted a moratorium of payment and the Purchaser has not been declared bankrupt and to its best knowledge no action or request is pending to declare the Purchaser bankrupt.
5. This Agreement has been duly authorised by the Purchaser and will constitute valid and legally binding obligations of the Purchaser enforceable against the Purchaser in accordance with its terms, except as such enforceability may be limited by applicable bankruptcy, insolvency, reorganisation or other similar laws affecting the enforcement of the creditors' rights generally or by general principles of equity.
6. The (class A) ordinary shares in the capital of the Purchaser shall be validly issued and placed, fully paid up and free and clear of Encumbrances. The uniQure DRs and the (class B) ordinary shares in the capital of the Purchaser underlying the uniQure DRs shall be validly issued and placed, fully paid up and free and clear of Encumbrances.

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## SCHEDULE 5 — SELLER WARRANTIES

1. Each of the Subsidiaries is duly organised and validly existing as a *besloten vennootschap met beperkte aansprakelijkheid* under the laws of the Netherlands, each of the Subsidiaries has the corporate power to carry on its business as presently conducted and each of the Subsidiaries has all requisite power to enter into the Agreement.
2. Each of the Subsidiaries is duly registered with the trade register held by the competent Chamber of Commerce.
3. Neither of the Subsidiaries has been dissolved and no resolution to dissolve either of the Subsidiaries has been adopted.
4. Neither of the Subsidiaries has requested or been granted a moratorium of payment and neither of the Subsidiaries has been declared bankrupt and to the Seller's best knowledge no action or request is pending to declare either of the Subsidiaries bankrupt.
5. The Sale Shares have been validly issued and placed. The Sale Shares are free and clear of Encumbrances. There are no depositary receipts (*certificaten van aandelen*) of the Sale Shares in existence. The Seller is the sole and exclusive legal and beneficial owner of the Sale Shares.
6. The Seller has not granted any rights to acquire shares in the capital of the Subsidiaries through subscription, conversion or otherwise.
7. The Seller has no actual or contingent obligation to transfer any of the Sale Shares to a third party (with the exception of the obligations of Seller to Purchaser arising out of this Agreement), or to create any Encumbrance in respect of the Sale Shares.
8. The Seller has full right, power and authority to sell and transfer the Sale Shares.
9. The Seller is duly organised and validly existing as a *naamloze vennootschap* under the laws of the Netherlands and has all requisite power to enter into this Agreement.
10. This Agreement has been duly authorised by the Seller and each of the Subsidiaries and on the Completion Date will constitute valid and legally binding obligations of the Seller and each of the Subsidiaries enforceable against them in accordance with its terms, except as such enforceability may be limited by applicable bankruptcy, insolvency, reorganisation or other similar laws affecting the enforcement of the creditors' rights generally or by general principles of equity.

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## SCHEDULE 6 — PROJECT KAIROS TERM SHEET

3

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Stibbe

Amsterdam Brussels Luxembourg London New York Dubai

STRICTLY CONFIDENTIAL

TERM SHEET  
PROJECT UNIQUIRE

This term sheet (the **Term Sheet**) summarizes the principal proposed terms of the cooperation among (i) Forbion Co-Investment II Coöperatief U.A., Coöperatieve AAC LS U.A. and Forbion Co-Investment Coöperatief U.A. (together referred to as **Forbion**) and (ii) uniQure B.V. (**uniQure**, or the **Company**) as the company.

uniQure intends to acquire the assets and liabilities of Amsterdam Molecular Therapeutics (AMT) Holding N.V. (**AMT** together with Forbion and uniQure: the **Parties**, or each: a **Party**), in exchange for depositary receipts (each: a **DR** and any holder of a DR: a **DR Holder**) for ordinary shares issued by a foundation (*stichting administratiekantoor*) holding ordinary shares in the capital of uniQure (**STAK**) (the **AMT Transaction**) pursuant to a business acquisition agreement



(the **BAA**), to be entered into between, *inter alia*, uniQure and AMT. Upon completion of the AMT Transaction (the **BAA Completion**), AMT will be dissolved and the DRs will be distributed to the AMT shareholders, whereupon holders of shares representing 5% or more of the issued and outstanding share capital of AMT immediately following BAA Completion (together with Forbion: the **Investors**, or each: an **Investor**) will be offered to exchange their DRs for shares in the capital of uniQure (the **Exchange Offer**).

Now, the Parties agree that (i) a shareholders' agreement in respect of uniQure, as well as (ii) ancillary documentation such as (a) the articles of association (*statuten*) of uniQure, (b) articles of incorporation of STAK, (c) the terms and conditions (*administratievoorwaarden*) of STAK, and (d) related documentation (all together, the **Governing Documents**) shall be entered into or, as the case may be, come into full force and effect immediately prior to BAA Completion. Upon BAA Completion, also completion under the Governing Documents (**Completion**) shall occur. The Parties agree that the documentation referred to above shall reflect the terms and conditions of this Term Sheet and shall furthermore be negotiated in good faith prior to Completion.

The proposed subject matter of this Term Sheet is specifically subject to the conditions set forth herein.

## 1 CHRONOS TRANSACTION

### 1.1 General principles

- uniQure and AMT N.V. (**AMT**) negotiate the BAA for the acquisition by uniQure of the assets and liabilities of AMT.
- AMT must convocate a general meeting of shareholders in order to obtain shareholder approval for the AMT Transaction. The timing of the shareholders meeting is 42 days after the convocation. Together with the convocation, the BAA and a summary thereof and of the other terms of the AMT Transaction will be circulated to the AMT shareholders in a shareholders circular (the **Shareholders Circular**). The Shareholders Circular shall be prepared by AMT and uniQure jointly.

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- In consideration of the assets and liabilities to be acquired by uniQure, uniQure shall issue a number of DRs, equal to the aggregate number of shares in the capital of AMT issued and outstanding at the time of the BAA Completion.
  - Forbion shall convert its convertible loan (with a face value of EUR 5,000,000) together with accrued interest immediately after BAA Completion at an agreed (and revised) conversion price of EUR 1 per share.
  - uniQure shall procure that the DRs so distributed to AMT will be accepted for clearing and trading through the clearing systems of Euroclear Nederland. The DRs shall be distributed to the AMT shareholders upon the shareholders meeting of AMT having resolved to liquidate AMT. Pursuant to the Governing Documents, the shares held by STAK will be subject to a drag right by the requisite number of Investors.
  - The distribution to AMT shareholders shall not be deemed an 'offer of securities' under the terms of the Financial Supervision Act (Wft) and consequently in respect of such distribution AMT (or uniQure) will not be required to offer a prospectus).
  - uniQure will offer each holder of DRs representing 5% or more of the issued and outstanding share capital of AMT immediately following BAA Completion to exchange its DRs in ordinary shares in the capital of uniQure (the Exchange Offer). As the Exchange Offer will be made to less than 100 persons, uniQure is exempted from issuing a prospectus in respect of the Exchange Offer.
  - AMT' shareholders meeting shall resolve to dissolve AMT, with the DRs to be distributed in the context of such dissolution.

### 1.2 Business purchase agreement and conditions precedent

The BAA shall be subject to, *inter alia*, the following conditions precedent:

- (i) the approval of AMT' shareholders meeting of the AMT Transaction; and
- (ii) an irrevocable equity funding of at least EUR 1 million by a new investor (the **New Investor**), at terms acceptable to uniQure; and
- (iii) no material adverse change will occur in the profitability, financial or trading position or the prospects of AMT and/or any of its subsidiaries; and
- (iv) Senter Novem has agreed with a transfer of all existing contracts with AMT to uniQure.

### 1.3 Public announcements

Regulatory rules require AMT to make public announcements through a press release, *inter alia*, immediately upon occurrence of the following events:

- shareholders approval of the AMT Transaction;
- execution of the BAA; and

- 
- BAA Completion.

## 2 UNIQUE

- (A) Corporate structure, Capitalization and new Funding

(1)	<i>Company</i>	uniQure B.V., a newly incorporated Dutch private limited liability company ( <i>besloten vennootschap met beperkte aansprakelijkheid</i> ).
(2)	<i>Capitalization (authorized capital)</i>	Different classes of ordinary shares (with a nominal value of EUR 0.01 each) ( <b>Ordinary Shares</b> ), to allow each Investor to hold Ordinary Shares of a separate class, with Investors holding Ordinary Shares Class A and STAK holding Ordinary Shares Class B.
(3)	<i>New Financing</i>	Forbion to commit to an investment in an amount of EUR 6 million and New Investor to commit to an irrevocable equity funding in an amount of at least EUR 1 million, to be effected by issuing Ordinary Shares Class A at a price per Ordinary Share of EUR 0.614 each (the <b>New Investment</b> ).
(4)	<i>Anti-dilution Protection</i>	<p>Ordinary Shares Class A issued to Forbion and the New Investor pursuant to the New Investment will be entitled to full ratchet anti-dilution rights in subsequent rounds where the per share consideration received by the Company is less than the per share consideration paid by Forbion and the New Investor pursuant to the New Investment. These anti-dilution rights shall be subject to carve-outs for shares issued to employees, consultants and directors. In such case, new shares in the Company are to be issued either with payment to be made from the Company's share premium reserve, or if not available, such new shares are to be issued at par value and transferred to Forbion and the New Investor, with the number of shares computed according to the following formula:</p> $(P1 - P2) / P2 \times Q$ <p>Whereas:</p> <p>(i) P1 to mean the issue price paid by Forbion and the New Investor pursuant to the New Investment;</p> <p>(ii) P2 to mean the issue price offered in the subsequent capital increase.</p>

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		Q to mean the number of shares subscribed by Forbion and the New Investor pursuant to the New Investment.
(5)	<i>Capitalization (issued capital)</i>	Upon BAA Completion (post conversion of the Forbion convertible loan, as set out above), full acceptance of the Exchange Offer and completion of the New Investment, the issued capital of uniQure shall be held as set out in Schedule 3.
(6)	<i>STAK</i>	<i>Stichting Administratiekantoor</i> , holder of the Ordinary Shares Class B and issuer of DRs (without the Company's cooperation) ( <i>certificaten</i> ) to certain eligible investors in AMT (the DR Holders), entitling the DR Holders to the distribution of the profits of uniQure relating to the Ordinary Shares held by STAK, for each DR Holder in proportion to the number of DRs that they hold. DR Holders shall be entitled to instruct the STAK management board how to vote on the shares underlying the DRs they hold in a general meeting of shareholders of uniQure. The STAK management board shall vote shares for which no instruction has been given by the DR holder entitled thereto.
(7)	<i>STAK management board</i>	2 DR representatives and 1 independent member.

**(B) Governance**

No.	Subject	Term
(8)	<i>Management board</i>	The management board of uniQure (the <b>MB</b> ) shall initially consist of the directors currently constituting the AMT' management board, i.e. Mr. J. Aldag and Mr. P.J. Morgan (employment conditions subject to review by Forbion). Any AMT stock option plan currently in force and applicable to Mr. J. Aldag and Mr. P.J. Morgan will terminate on BAA Completion and will be replaced by a new uniQure stock option plan. Prior to BAA completion, Mr. J. Aldag and Mr. P.J. Morgan will be required to waive any rights they may be entitled to under any current existing AMT stock option plan.
(9)	<i>Supervisory board</i>	The supervisory board of uniQure (the <b>SB</b> ) shall initially consist of 3 investor representatives (the <b>Investor Representatives</b> ) and 4 other members, i.e. S.J.H. van Deventer, H.A. Slootweg and a person nominated in accordance with Schedule 1, as the Investor Representatives, and J.M. Feczko, P.M.M.J. van Holle, F. Meyer and a newly to be appointed independent member, as the independent members.

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(10)	<i>Supervisory board committees</i>	The SB shall have at least two standing committees, i.e. the audit committee and the remuneration and appointment committee, to be appointed by the SB from its own members. Each committee shall consist of 3 members, of which 1 Investor Representative.
(11)	<i>Governance (Management Board and Supervisory Board)</i>	Schedule 1 sets out specific governance provisions in relation to uniQure relating to Management Board and Supervisory Board resolutions.
(12)	<i>Governance (General Meeting)</i>	Schedule 2 sets out specific governance provisions in relation to uniQure relating to resolutions of the

(13)	<i>ESOP</i>	To provide further incentives to employees, directors and or outside consultants and advisors, contemporary with completion of the AMT Transaction, the shareholders of the Company will authorise a new unallocated pool of options and / or warrants for future grants to employees, directors and / or outside consultants and advisors, representing 15% of the fully diluted share capital of the Company post BAA Completion, with (i) a vesting period of three years; 1/3 of the options to be vested after one year, the other 2/3 to be vested in year 2 and 3 on a pro rata — i.e. linear — basis; and (ii) the exercise price of the options being the price paid by Forbion. The SB shall be authorised to grant options or warrants within the scheme. A resolution of the SB to this effect requires the positive vote of at least two Investor Representatives.
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(C) Transfers of Shares

No.	Subject	Term
(14)	<i>Transfer of Ordinary Shares</i>	Each transfer of Ordinary Shares (be it to an Investor's affiliate or group company, a Portfolio Transfer, a Sale or otherwise) is always subject to the condition that the purchaser of such transferred Ordinary Shares is or becomes bound to the Governing Documents.
(15)	<i>Transfers of DRs</i>	DRs are freely transferable, subject to restrictions under applicable laws.
(16)	<i>Portfolio Transfer</i>	Subject to the requirement for a transferee to sign a deed of adherence, the Ordinary Shares shall at all times be freely transferable (other than to competitors of the Group) as part of a transfer of an Investor's investment portfolio (in whole or in part) or to entities in which an Investor has an economic interest ( <b>Portfolio Transfer</b> ).

(17)	<i>Right of first offer other Investors</i>	If an Investor intends to sell (the <b>Selling Investor</b> ) any Ordinary Shares other than in a Portfolio Transfer, or transfers to affiliates, the following shall apply. The Selling Investor shall notify the other Investors of its wish to sell Ordinary Shares and shall invite the other Investors to make an offer, on the basis of a minimum price, for these Ordinary Shares within 2 weeks from receipt of the notice. The Ordinary Shares offered shall be allocated to the other Investors that have made an offer for at least the minimum price for the price offered by the relevant Investors. If the other Investors have not subscribed for all Ordinary Shares offered for sale by the Selling Investor, the Selling Investor is free to offer those shares to any third party for a price at least equal to the minimum price stated in the notice to the other Investors.
(18)	<i>Good and Bad Leaver</i>	Equity interests to be granted to employees, directors and / or outside consultants and advisors pursuant to the employee stock option plan shall be made subject to good- and bad leaver arrangements pursuant where to the relevant person will be required to offer their interests to (a person designated by) the Company in case such person's employment (or other) contract with the Company (or a group company of the Company) terminates.
(19)	<i>Tag offer</i>	If Investors holding 51% or more of the issued and outstanding Ordinary Shares Class A propose to sell Ordinary Shares to a third party (a <b>Sale</b> ), each other holder of Ordinary Shares will have the right (as a condition to such Sale) to require that third party purchaser to purchase a pro rata portion of such holder's Ordinary Shares (a <b>Tag Offer</b> ) to be completed simultaneously with the Sale.
(20)	<i>Drag offer</i>	In case of a Sale, the proposed seller or sellers may, upon agreement of the terms and conditions of a bona fide offer by a third party purchaser for one or more of its or their Ordinary Shares (a <b>Drag Offer</b> ) require each (other) holder of Ordinary Shares to transfer all (but not less than all) of such shares (the <b>Drag Shares</b> ) on the same terms and conditions as those offered in the Drag.

(D) Exit

No.	Subject	Term
(21)	<i>Intention to Exit</i>	It is the intention of the Investors, that an Asset Sale or an Exit be achieved as soon as practically possible and commercially sensible.

(22)	<i>Nature of an Exit</i>	<p>An <b>Exit</b> shall be:</p> <ul style="list-style-type: none"> <li>· a transfer (or a series of related transfers) of all the Ordinary Shares issued (other than as a result of a transfer by an Investor to an affiliate);</li> <li>· the listing and admission to trading on a market for listed securities of either (i) the Company's shares, (ii) an intermediate holding company's shares or (iii) the shares of new holding company established for the purposes of the Listing (a <b>Listing</b>); or</li> <li>· a distribution pursuant to a winding-up or dissolution of the Company or any holding company of the Company, including following an Asset Sale.</li> </ul>
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		An <b>Asset Sale</b> shall be a sale by one or more Group Companies of all, or substantially all, of the Group's business, assets and undertaking.
(23)	<i>Appointment of Advisors</i>	Investors holding 51% or more of the issued and outstanding Ordinary Shares Class A and the Company shall jointly decide on the appointment by the Company of its advisors in connection with the Exit.
(24)	<i>Obligations on an Exit or an Asset Sale</i>	The Company shall co-operate and take such steps as reasonably required (taking into account their rights and obligations under this term sheet) in respect of any proposed Exit or Asset Sale subject always to fiduciary duties and compliance with applicable law.

(E) Miscellaneous

No.	Subject	Term
(25)	<i>Provision of Information</i>	<p>The Company shall be required to supply to the Investors:</p> <ul style="list-style-type: none"> <li>at least 30 business days prior to the start of the new financial year, a draft annual budget (for comment) including profit and loss projections, monthly cash flow projections and balance sheet projections including line items on total proposed (i) capital expenditure, (ii) project financing and (iii) total costs of other investments for comments by the Investors;</li> <li>within 30 days after the start of the relevant financial year, the annual budget per the above specifications;</li> <li>within 15 business days after the end of each month, a monthly information package to include (i) updated</li> </ul>

		<p>liquidity forecast (12 months, in aggregate and breakdown per major project) and reconciliation with previous month, (ii) qualitative comments highlighting development progress, financing events and sales process, and (iii) progress reporting on 5 biggest projects (format to be acceptable to Investors holding 51% or more of the issued and outstanding Ordinary Shares Class A, but in any case shall include executive summary on key elements (progress, costs, liquidity against budget etc));</p> <ul style="list-style-type: none"> <li>within 20 business days after the end of each quarter, a quarterly information package to include (i) monthly information, (ii) updated format revised business plan; (iii) a reconciliation with previous quarterly revised business plan; (iv) progress reporting on 5 biggest projects (format to be acceptable to Investors holding 51% or more of the issued and outstanding Ordinary Shares Class A (acting reasonably) but in any case shall include: (a) executive summary on key elements (progress, costs, liquidity against budget, project financing, general economical climate etc) and (b) project monitor summary if project has commenced (to be delivered in the same format as required by the financiers of the relevant project, if available) and (v) reporting on financing (format to be acceptable to Investors holding 51% or more of the issued and outstanding Ordinary Shares Class A (acting reasonably) but shall in any case include regularity of all financings);</li> <li>as soon as they become available, copies of annual valuations of underlying assets; and</li> <li>other information reasonably required by Investors holding 51% or more of the issued and outstanding Ordinary Shares Class A.</li> </ul> <p>The format of the reporting shall be designed by the Company and shall be in form and substance satisfactory to Investors holding 51% or more of the issued and outstanding Ordinary Shares Class A.</p>
(26)	<i>STAK Information Rights</i>	<ul style="list-style-type: none"> <li>Within 20 business days after a general meeting of shareholders of the Company, the DR Holders shall be entitled to receive from the STAK management board a copy of the adopted annual accounts and a list of resolutions adopted by the general meeting of shareholders.</li> <li>Within 20 business days after the end of each quarter, the DR Holders shall be entitled to receive from the STAK management board a quarterly financial statement, in a format</li> </ul>

		<p>to be approved by the SB.</p> <ul style="list-style-type: none"> <li>There shall be no obligation to make the information as referred to in this section 0 publicly available by placing it on the Company's or any other website.</li> </ul>
(27)	<i>Compensation of costs</i>	The Company and / or AMT shall bear the costs reasonably incurred (including, for the avoidance of doubt, legal fees) by Forbion in connection with the AMT Transaction in the event that (i) BAA Completion occurs or (ii) the BAA is terminated as a result whereof and pursuant to the BAA, the Company will be paid a break fee.
(28)	<i>Confidentiality</i>	Investors will be subject to customary restrictions on the use and disclosure of confidential information.

(29)	Accounting regime	Dutch GAAP will apply. The audit committee will be authorised to adopt IFRS as the applicable accounting standard.
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(30)	Governing law	This term sheet is governed by Dutch law. The definitive documents will be governed by Dutch law.
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[signature page to follow]

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Signed and agreed on 16-2-2012.

/s/ H.A. Slootweg  
uniQure B.V.  
By: Forbion 1 Co II Management B.V.  
By: H.A. Slootweg  
Director  
Date: 16-2-2012

/s/ M.A. van Osch  
uniQure B.V.  
By: Forbion 1 Co II Management BV  
By: M.A. van Osch  
director  
Date: 16-2-12

/s/ H.A. Slootweg  
Forbion Co-Investment II Coöperatief U.A.  
By: Forbion 1 Co II Management B.V.  
By: H.A. Slootweg  
Director  
Date: 16-2-2012

/s/ M.A. Jan Osch  
Forbion Co-Investment II Coöperatief U.A.  
By: M.A. van Osch  
director  
Date: 16-2-2012

/s/ H.A. Slootweg  
Coöperatieve AAC LS U.A.  
By: Forbion 1 Management B.V.  
By: H.A. Slootweg  
Director  
Date: 16-2-2012

/s/ M.A. van Osch  
Coöperatieve AAC LS U.A.  
By: Forbion 1 Management BV  
By: M.A. van Osch  
director  
Date: 16-2-2012

/s/ H.A. Slootweg  
Forbion Co-Investment II Coöperatief U.A.  
By: Forbion 1 Management B.V.  
By: H.A. Slootweg  
Director  
Date: 16-2-2012

/s/ M.A. van Osch  
Forbion Co-Investment II Coöperatief U.A.  
By: Forbion 1 Management BV  
By: M.A. van Osch  
director  
Date: 16-2-2012

Amsterdam Molecular Therapeutics (AMT) Holding N.V.  
By: /s/ Jörn Aldag  
Date: 16.02.2012

Amsterdam Molecular Therapeutics (AMT) Holding N.V.  
By: /s/ PJ Morgan  
PJ Morgan  
Date: CFO

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## SCHEDULE 1: GOVERNANCE (MANAGEMENT BOARD AND SUPERVISORY BOARD)

This schedule and the allocation in respect of reserved matters assumes the following starting points:

1. The Company shall have a Management Board and a Supervisory Board.
2. The Management Board shall consist of 2 or more members appointed by the general meeting of shareholders of the Company (the **General Meeting**) in accordance with a binding nomination by any Investor or group of Investors holding at least 51% of the Ordinary Shares Class A.
3. The Supervisory Board shall consist of 7 directors, of whom 3 directors (one of whom shall be the chairman of the Supervisory Board (the **Chairman**)) shall be appointed by the General Meeting in accordance with a binding nomination by any Investor or group of Investors holding at least 51% of the Ordinary Shares Class A. Prior to the making of a nomination the Investors shall consult with the other Investors and STAK about the identity and the qualifications of such person included on a shortlist of potential nominees and the Investors shall take any substantiated objections against potential nominees into account in making their decision to formally nominate such person. In case of a deadlock of votes in a meeting of the Supervisory Board, the Chairman will have a casting vote.
4. The Supervisory Board shall meet at least 6 times per year (or such other number as the Chairman may require) in person at scheduled meetings and so often as required for the proper fulfilment of the role of the Supervisory Board, either in person or by conference call.
5. The Management Board will be responsible for all operational matters in respect of the Company and its subsidiaries (collectively: the **Group** and any member of the Group also: a **Group Company**) but will require the prior approval of the Supervisory Board acting by simple majority for the matters listed

in Part A of this Schedule.

6. The items listed in Parts B and C of this Schedule will require approval by or the adoption of a prior resolution of Investors holding at least 51% or 66<sup>2</sup>/<sub>3</sub>%, respectively, of Class A Ordinary Shares held by Investors.

## **Part A**

### **Supervisory Board approval**

- (a) Any amendment of articles of association of any Group Company.
- (b) Voting on shares or similar equity interests in a Group Company (which provision would need to be mirrored in articles of association of Group Companies), for resolutions mentioned in Part A, B and C.
- (c) The instigation or the settlement of any material litigation or arbitration or mediation proceedings by a Group Company, for the purpose of which 'material' shall mean an interest or claim that is of strategic importance to the Group or has a monetary value of at least EUR 100,000.

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- (d) Any proposals to the General Meeting to materially change the emoluments of members of the Management Board, including bonuses and option schemes.
- (e) The removal or appointment of the auditors of any Group Company, other than the reappointment of existing auditors.
- (f) Remuneration of the auditors of the Company.
- (g) Approval of any change in accounting policies of any Group Company.
- (h) Alteration to the financial year end of any Group Company.
- (i) Non-project related capital expenditure exceeding in one transaction or event or a series of related transactions or events, in excess of an amount of EUR 50,000 but less than EUR 100,000, which is not included in an approved business plan or budget.

## **Part B**

### **Approval with Simple Majority Investor Consent (51%) of Class A Ordinary Shares held by Investors**

- (a) Unless specified in an approved business plan of the Company, entering into or materially changing borrowing and lending arrangements (including issuance of debt instruments) by any Group Company, exceeding an amount of EUR 250,000.
- (b) Unless specified in an approved business plan of the Company, establishing/closing any material branch, establishment, agency or business of any Group Company.
- (c) Unless specified in an approved business plan of the Company, entering into any material joint venture, partnership or profit sharing arrangement or licensing agreement by any Group Company.
- (d) Unless specified in an approved business plan of the Company, the expansion or development of the Group or any of its business other than through a Group Company.
- (e) Adoption of or amendment to the current business plan (to be in agreed form) and budget (to be in agreed form).
- (f) Creation or release of any security or (save in the ordinary course of trading and consistent with past practice) granting of guarantees by any Group Company, exceeding an amount of EUR 250,000.
- (g) Unless specified in an approved business plan of the Company, any material acquisitions or disposals by any Group Company.
- (h) The appointment or removal of any member of the Supervisory Board or Managing Director of a Group Company other than the Company.

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- (i) Non-project related capital expenditure exceeding in one transaction or event or a series of related transactions or events, in an amount of EUR 100,000 or more, which is not included in an approved business plan or budget.
- (j) Establishment and material amendment of any management incentive scheme of any Group Company (other than the Company).

## **Part C**

### **Approval with Qualified Majority Investor Consent (66 2/3%) of Class A Ordinary Shares held by Investors**

- (a) Any change in a Group Company's (other than the Company's) share capital.
- (b) Unless specified in an approved business plan of the Company, any material change of the nature or the name of the business of the Group.
- (c) Entry into, termination or variation of any contract or arrangement by a Group Company with an Investor, other than financing arrangements.

- (d) Any distribution from reserves (other than wholly intra-group) by any Group Company.
- (e) Transactions by a Group Company outside of its ordinary course.
- (f) Taking steps to commence insolvency or winding-up proceedings of a Group Company (including the application for suspension of payment of debts by a Group Company).

## SCHEDULE 2: GOVERNANCE (SHAREHOLDERS MEETING)

### Part A

#### Approval with Qualified Shareholder Consent

The following resolutions of the General Meeting will require the affirmative vote of Shareholders holding at least 66<sup>2</sup>/<sub>3</sub>% of Ordinary Shares.

#### Approval with Qualified Majority Shareholder Consent (66 2/3%) of Ordinary Shares

- a) The merger (*fusie*) or demerger (*splitsing*) of the Company.
- b) The initiation of liquidation or dissolution of the Company or approve the filing for bankruptcy.
- c) The amendment of the articles of association of the Company.
- d) Appointment or dismissal of the Company's auditors.

### Part B

#### Approval with Simple Majority Investor Consent (51%) of Class A Ordinary Shares held by Investors

The following resolutions of the General Meeting will require the affirmative vote of Investors holding at least 51% of Class A Ordinary Shares.

- a) The issue of new equity securities (including options and warrants).
- b) The exclusion or restriction of pre-emptive rights with respect to the issue of new equity securities.
- c) The redemption (*intrekking*) or the reduction of the nominal value of any shares.
- d) The purchase (*inkoop*) by the Company of shares in its own capital, shares in the capital of any subsidiary, or depositary receipts (*certificaten van aandelen*) representing any such shares (whether or not issued "with the co-operation of the Company").
- e) The declaration of dividends or distributions.
- f) The delegation of powers with respect to the issue of securities, the exclusion of pre-emptive rights, or the approval of the purchase of the Company's own shares.
- g) Determination or variation of the remuneration of members of the Management Board and of the Supervisory Board.

## SCHEDULE 3: ISSUED CAPITAL IMMEDIATELY UPON FULL ACCEPTANCE OF THE EXCHANGE OFFER

## SCHEDULE 7 – PRESS RELEASE



## Extraordinary Shareholder Meeting called to vote on the Transaction

**Amsterdam, The Netherlands — February 17, 2012** — Amsterdam **Molecular** Therapeutics (Euronext: AMT) announced today that its board of directors is recommending a substantial corporate restructuring and financing transaction which, if approved by shareholders, will result in the assets and certain liabilities being acquired by a newly formed private company, uniQure BV, and the AMT legal entity being liquidated and delisted.

This transaction will:

- support the future funding of AMT's current gene therapy development pipeline;
- reduce operating costs; and
- enable disposal of current loan note obligations.

Consequently, management believes the uniQure transaction will allow **AMT's** shareholders to benefit from the future potential value in the business. This includes, if successful, completion of the ongoing collaboration discussions for the hemophilia B program and further collaborations on the GDNF program.

AMT has entered into a definitive agreement with Amsterdam-based uniQure to acquire the assets and certain liabilities of AMT in return for unlisted uniQure depository receipts ("DRs") which may be exchangeable for uniQure shares as described further below. There is no cash component to the consideration. The proposed transaction has been evaluated by a special committee of the board ("Special Committee") together with Ernst & Young, which has advised the Special Committee that the Transaction is fair and reasonable so far as AMT shareholders are concerned. The disposal of the company's programs, assets and certain liabilities has been unanimously approved by the board of supervisory directors, which recommends that AMT shareholders adopt the resolution at an Extraordinary Shareholder Meeting on March 30, 2012.

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On completion, uniQure will receive additional equity funding of € 7.0 million, including € 6.0 million from Forbion Capital managed funds together with € 1.0 million in additional new financing to be secured by AMT prior to completion. In addition, uniQure will take over AMT's liability related to the € 5.0 million convertible loan notes and accrued interest of € 0.3 million. AMT will receive one new uniQure DR for every existing issued and outstanding AMT share. AMT will subsequently be dissolved and, as an advance liquidation payment, the uniQure DRs shall be distributed to AMT's shareholders. The uniQure DRs shall not be listed.

"We believe the proposed transaction will ensure the future of AMT following the failure to gain approval of Glybera in 2011 given the very limited options available to us. While we have pursued various avenues to raise additional funds on the open capital markets, worked diligently to engage in partnering discussions and have cut personnel, programs and spending down drastically, we are ultimately still facing a very precarious financial position with a cash reach to early April," explained Jörn Aldag, CEO of AMT. "The Transaction offers the only viable way to secure a capital injection, the ability to better focus resources towards the advancement of our gene therapy pipeline and also concluding collaborations which validate our platform such as a hemophilia B partnership. We ultimately believe that this transaction offers the renewed possibility of a meaningful exit for our shareholders, allowing them to benefit from the potential future upside in the business, including the possible outcome of the reconsideration by CHMP of our Glybera product."

On the basis that the Transaction is in the best interests of AMT, its stakeholders, and the business, the Special Committee supports the Transaction and shall recommend the Extraordinary General Meeting of shareholders ("EGM") to be held on March 30, 2012 to approve the Transaction.

uniQure will continue with AMT's strategy to invest in the hemophilia B, GDNF and AIP programs, as well as the collaboration with Institut Pasteur on Sanfilippo B, but will not invest significant additional funds into Glybera unless and until it receives a positive decision from regulatory authorities. All employees of AMT will continue in employment with uniQure on the same terms. The members of the Supervisory and Management Boards will also transfer to uniQure, with the exception of Mr. Ferdinand Verdonck, who chairs the Special Committee. Further terms of the Agreement and information on uniQure are set out below in a circular to Shareholders ("Shareholder Circular"), copies of which can be obtained free of charge from the Company's office and at the Company's website ([www.amtbiopharma.com](http://www.amtbiopharma.com)).

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### New funding, conversion of convertible loan notes and further information on uniQure

UniQure has been specifically created for the Transaction and financing, and has no other assets or liabilities.

Upon completion, Forbion Capital managed funds will subscribe € 6.0 million in new equity for 9,771,987 ordinary shares in uniQure at an issue price of €0.614 per share, being the mean closing share price of AMT on NYSE Euronext in Amsterdam for the five business days prior to the date the Business Acquisition Agreement was entered into. This additional financing from Forbion funds is conditional on AMT securing an additional € 1.0 million in new equity funding on the same terms from other sources. Together, the € 7.0 million in new financing will secure the immediate future of the AMT business and is expected to enable the successful conclusion of the current partnering negotiations relating to the hemophilia B program. The majority of this new Forbion funding comes from funds that are only permitted to invest in unlisted companies and this money is therefore not available to AMT directly.

Following completion, the transferred liabilities relating to the convertible loan notes shall be converted to 5,320,000 ordinary shares in uniQure using a conversion price of € 1.00 per share. Following the Transaction Forbion will be the largest shareholder in uniQure with significant rights relating to the conduct and governance of the business.

Each AMT shareholder that on the Distribution Record Date holds at least 1.555.054 shares in AMT and that will hence receive at least 1.555.054 DRs shall be entitled to exchange its DRs for an equal number of ordinary shares in uniQure. Each AMT shareholder with fewer AMT shares shall receive the equivalent number of DRs but shall not be entitled to exchange its DRs for an equal number of ordinary shares in uniQure.

uniQure will be subject to certain terms and conditions relating to its governance and operation; these are described further in the Shareholder Circular.



An EGM has been convened for March 30, 2012 to approve the Transaction and resolve on certain related matters, as set out in the agenda and the explanatory notes thereto, which are available through the company's website.

Forbion has committed to vote in favour of the Transaction in respect of the 19.4% of AMT's shares it controls.

#### Shareholder information meetings

AMT will hold two information meetings for shareholders at its offices on February 23, 2012 and March [20], 2012. Further details will be posted on the AMT website.

#### About Amsterdam Molecular Therapeutics

AMT is a world leader in the developing of human gene based therapies. AMT has a product pipeline of gene therapy products in development for hemophilia B, acute intermittent porphyria, Parkinson's disease and Sanfilippo. Using adeno-associated viral (AAV) derived vectors as the delivery vehicle of choice for therapeutic genes, the company has been able to design and validate probably the world's first stable and scalable AAV manufacturing platform. This proprietary platform can be applied to a large number of rare (orphan) diseases caused by one faulty gene and allows AMT to pursue its strategy of focusing on this sector of the industry. AMT was founded in 1998 and is based in Amsterdam. Further information can be found at [www.amtbiopharma.com](http://www.amtbiopharma.com).

#### About uniQure

uniQure BV is a private company created specifically for the Transaction. It is funded by Forbion Capital Partners, an existing investor in AMT. uniQure will act as the new holding company for the gene therapy business currently carried out by AMT.

For further enquiries:

Jörn Aldag	Mike Sinclair	Sander Slootweg
CEO	Partner	General Partner
AMT	Halsin Partners	Forbion Capital
Partners		

Tel: +31 20 566 8014  
[j.aldag@amtbiopharma.com](mailto:j.aldag@amtbiopharma.com)

Tel: +44 20 7318 2955  
[msinclair@halsin.com](mailto:msinclair@halsin.com)  
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*Certain statements in this press release are "forward-looking statements" including those that refer to management's plans and expectations for future operations, prospects, and financial condition. Words such as "strategy," "expects," "plans," "anticipates," "believes," "will," "continues," "estimates," "intends," "projects," "goals," "targets," and other words of similar meaning are intended to identify such forward-looking statements. Such statements are based on the current expectations of the management of AMT only. Undue reliance should not be placed on these statements because, by their nature, they are subject to known and unknown risks and can be affected by factors that are beyond the control of AMT. Actual results could differ materially from current expectations due to a number of factors and uncertainties affecting AMT's business. AMT expressly disclaims any intent or obligation to update any forward-looking statements herein except as required by law.*

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**The securities mentioned herein have not been and will not be registered under the U.S. Securities Act of 1933, as amended (the "Securities Act"), or under the securities laws of any state or other jurisdiction of the United States and may not be offered, sold, resold, taken up, exercised, renounced, transferred or delivered, directly, or indirectly, within the United States except pursuant to an applicable exemption from, or in a transaction not subject to, the registration requirements of the Securities Act. No public offering of the securities mentioned herein is being made in the United States or any other jurisdiction.**

**This announcement does not constitute or forms a part of any offer to solicitation to purchase or subscribe for securities in any jurisdiction or jurisdictions in which such**

**offers or sales are unlawful prior to registration or qualification under the securities laws of any such jurisdiction.**

**The materials contained herein have not been submitted to or reviewed by the US Securities and Exchange Commission (the "SEC") or any state securities commission, and neither the SEC nor any such state securities commission has (a) approved or disapproved, (b) passed upon the merits of fairness of, or (c) passed upon the adequacy or accuracy of the disclosure of any materials contained herein. Any representation to the contrary is a criminal offence in the United States.**

#### SCHEDULE 8— NOTICES

All announcements or notices to the Seller shall be sent to the following address or to the following fax number:

Name	:	Amsterdam Molecular Therapeutics (AMT) N.V.
Attn	:	Mr. Joern Aldag
Address	:	Meibergdreef 61, 1105 BA

Place of residence : Amsterdam  
Country : The Netherlands  
Email : j.aldag@amtbiopharma.com

With a copy to:

Name : Simmons & Simmons LLP  
Attn : Mr. Michiel Wurfbain  
Address : Claude Debussylaan 247, 1082 MC  
Place of residence : Amsterdam  
Country : The Netherlands  
Email : michiel.wurfbain@simmons-simmons.com

as long as the Seller does not give notice to the other Parties of any other address.

All announcements or notices to the Purchaser shall be sent to the following address or to the following fax number:

Name : uniQure B.V.  
Attn : Vincent van Houten  
Address : Gooimeer 2 35, 1411 DC  
Place of residence : Naarden  
Country : The Netherlands  
Email : Vincent.van.Houten@forbion.com

With a copy to:

Name : Stibbe  
Attn : Mr. Egbert Vroom  
Address : Strawinskylaan 2001, 1077 ZZ  
Place of residence : Amsterdam  
Country : The Netherlands  
Email : Egbert.vroom@stibbe.com

as long as the Seller does not give notice to the other Parties of any other address.

All announcements or notices to AMT BV and/or AMT IP BV shall be sent to the following address or to the following fax number:

Name : Amsterdam Molecular Therapeutics (AMT) B.V. or Amsterdam Molecular Therapeutics (AMT)IP B.V.  
Attn : Mr. Joern Aldag  
Address : Meibergdreef 61, 1105 BA  
Place of residence : Amsterdam  
Country : The Netherlands  
Email : j.aldag@amtbiopharma.com

With a copy to:

Name : Simmons & Simmons LLP  
Attn : Mr. Michiel Wurfbain  
Address : Claude Debussylaan 247, 1082 MC  
Place of residence : Amsterdam  
Country : The Netherlands  
Email : michiel.wurfbain@simmons-simmons.com

as long as AMT BV and/or AMT IP BV does not give notice to the other Parties of any other address.

All announcements or notices to the Investor shall be sent to the following address or to the following fax number:

Name : Forbion Capital Partners  
Attn : Vincent van Houten  
Address : Gooimeer 2 35, 1411 DC  
Place of residence : Naarden  
Country : The Netherlands  
Email : Vincent.van.Houten@forbion.com

With a copy to:

Name : Stibbe  
Attn : Mr. Egbert Vroom  
Address : Strawinskylaan 2001, 1077 ZZ  
Place of residence : Amsterdam  
Country : The Netherlands  
Email : Egbert.vroom@stibbe.com

as long as the Investor does not give notice to the other Parties of any other address.



DATED 5 APRIL 2012

**Amsterdam Molecular Therapeutics (AMT) B.V.**

and

**Amsterdam Molecular Therapeutics (AMT) IP B.V.**

as the Transferees

and

**Amsterdam Molecular Therapeutics (AMT) Holding N.V.**

as the Transferor

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**DEED OF ASSIGNMENT OF  
CERTAIN ASSETS AND  
LIABILITIES OF AMSTERDAM  
MOLECULAR THERAPEUTICS  
(AMT) HOLDING N.V.**

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Stibbe

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**THIS DEED** is made on 5 April 2012.

**BETWEEN:**

- (1) **Amsterdam Molecular Therapeutics (AMT) B.V.**, a company incorporated under the laws of the Netherlands with its corporate seat in Amsterdam, the Netherlands, registered with the trade register of the Chamber of Commerce with number 34275365;
- (2) **Amsterdam Molecular Therapeutics (AMT) IP B.V.**, a company incorporated under the laws of the Netherlands with its corporate seat in Amsterdam, the Netherlands, registered with the trade register of the Chamber of Commerce with number 34275369;

(**Amsterdam Molecular Therapeutics (AMT) B.V.** and **Amsterdam Molecular Therapeutics (AMT) IP B.V.** collectively also the “**Transferees**” and each individually a “**Transferee**”), and

- (3) **Amsterdam Molecular Therapeutics (AMT) Holding N.V.**, a company incorporated under the laws of the Netherlands with its corporate seat in Amsterdam, the Netherlands, registered with the trade register of the Chamber of Commerce with number 33301321 (the “**Transferor**”)

The parties to this Agreement are hereinafter collectively referred to as the “**Parties**” and individually as a “**Party**”.

## RECITALS:

- (1) Pursuant to an agreement dated 16 February 2012 (the “**Economic Ownership Transfer Agreement**”) the Transferor contributed the economic ownership (*economische eigendom*) of the Business (as defined in the Economic Ownership Transfer Agreement) to the Transferees subject to and upon the terms and conditions of the Economic Ownership Transfer Agreement.
- (2) The Transferor and the Transferees wish to transfer the legal title (*juridische eigendom*) to the Business to the Transferees as contemplated in the Economic Ownership Transfer Agreement by entering into this asset transfer agreement (the “**Deed of Assignment**”) and performing all other actions necessary to transfer the legal title to, and therefore the ownership of, the Business to the Transferees in accordance with Clause 2 of the Economic Ownership Transfer Agreement.
- (3) The conditions stipulated in Clause 3 of the Economic Ownership Transfer Agreement have been fulfilled or waived and parties have agreed that the legal title of the Business shall be transferred on the execution of this Deed of Assignment, but prior to the Completion Date.
- (4) Furthermore, the Transferor and the Transferees wish to transfer the legal title (*juridische eigendom*) and the economic ownership (*economische eigendom*) of the Seller Loans to the Transferees and to effectuate such transfer by entering into this Deed of Assignment and performing all other actions necessary to effectuate such transfer.
- (5) The Transferor has obtained all internal corporate approvals required for the execution and performance of this Deed of Assignment.

IT IS AGREED as follows:

## 1. INTERPRETATION

Definitions used in this Deed of Assignment have the same meaning as given to them in the Economic Ownership Transfer Agreement unless stated otherwise, and the provisions of Clause 1 (Interpretation) of the Economic Ownership Transfer Agreement shall apply to this Agreement.

## 2. TRANSFER OF OWNERSHIP

### 2.1. Agreement to transfer

- 2.1.1. Subject to the terms and conditions of this Deed of Assignment and the Economic Ownership Transfer Agreement, the Transferor hereby transfers and delivers the legal title to the Business to the Transferees and the Transferees hereby accept and assume from the Transferor the Business, including the Business Assets and the Business Liabilities, whereby:
- (A) the legal title to the Business Intellectual Property Rights is transferred to AMT IP BV; and
- (B) the legal title to the other Business Assets and Business Liabilities is transferred to AMT BV.
- 2.1.2. The Transferees and the Transferor acknowledge that to the extent the transfer and delivery of any of the Business Assets and/or Business Liabilities requires a deed (*akte*) this Deed of Assignment constitutes a deed of transfer for such Business Assets and Business Liabilities to take effect from the execution of this Deed of Assignment.
- 2.1.3. The transfer and delivery of the Business Assets and Business Liabilities shall further be effectuated as set out in Clause 2 of the Economic Ownership Transfer Agreement.
- 2.1.4. The legal title to the respective parts of the Business shall be transferred to AMT IP BV and AMT BV by way of a contribution in kind (*inbreng in natura*) on the ordinary shares in the capital of the Transferees held by the Transferor, without the issuance of new shares. The value of such contribution will be recorded as share premium (*agiotorting*) in the books of AMT IP BV and AMT BV.

### 2.2. Business Goodwill and Know How

The Business Goodwill and Know How are hereby transferred to the Transferees.

### 2.3. Business Intellectual Property Rights

The Business Intellectual Property Rights are hereby transferred to AMT IP BV. AMT IP BV shall register the registered Business Intellectual Property Rights in its name in all relevant registers. To the extent any of the Business Intellectual Property Rights consists of a license (“**Business Permit**”), such license shall, if permitted under the license, be transferred in accordance with the relevant requirements of such license and in accordance with Clause 2.6, simultaneously with the execution of this Deed of Assignment.

### 2.4. Business Assets

- 2.4.1. All Business Assets, including but not limited to ICT, inventory and equipment, business records, business stock and work in progress and all other Business Assets that are transferable by delivery (*bezitsverschaffing*), to the extent such Business Assets are not held by others for or on

behalf of the Transferor, are hereby transferred and delivered to the relevant Transferee by giving access to the places where these Business Assets are physically located and by having delivered to the relevant Transferee all keys and title documents and other evidence of ownership.

- 2.4.2. To the extent that any of the Business Assets that are mentioned in Clause 2.4.1 are held by others for or on behalf of the Transferor, such Business Assets are transferred and delivered to the relevant Transferee by a notice from the Transferor to the other parties, also on behalf of the relevant Transferee, instructing such party to hold such Business Assets for or on behalf of the relevant Transferee to take effect prior to the Completion Date. The Transferor shall send the relevant Transferee a copy of such notices.
- 2.4.3. To the extent any of the Business Assets consists of a license, such license shall, if permitted under the license, be transferred in accordance with the relevant requirements of such license and in accordance with Clause 2.6 simultaneously with the execution of this Deed of Assignment.

## **2.5. Business Liabilities**

The Business Liabilities are hereby transferred to the relevant Transferee in accordance with the terms and conditions of the Economic Ownership Transfer Agreement.

## **2.6. Contracts and Business Permits**

All Contracts and Business Permits are hereby transferred to the relevant Transferee in accordance with the relevant requirements of such Contract or Business Permit and the terms and conditions of the Economic Ownership Transfer Agreement.

## **2.7. Other assets and liabilities**

Notwithstanding the provisions of Clause 2.1 up to and including 2.6, the Transferor hereby transfers and delivers the legal title of all other existing assets, business assets, liabilities and/or contracts of the Transferor, if any, to AMT BV and AMT BV hereby acquire and accept from the Transferor all such other assets, including but not limited to cash, business assets, liabilities and/or contracts.

## **2.8. Excluded assets and liabilities**

The provisions of this Deed of Assignment do not extend to the Loan Notes, Convertible Loan Note Agreement, Sale Shares, Administration, rights and obligations pursuant to the BAA, the listing agreement between the Transferor and Euronext Amsterdam, the contract with the ENL agent and the payment agent, the liquidity provider agreement between the Transferor and Kempen & Co N.V., the D&O Insurance policy of the Transferor, any outstanding obligations under any stock option plan or other employee plan of the Transferor and any and all agreements the Transferor entered into with Senter Novem. This

Deed of Assignment does therefore not constitute a transfer of the legal title to any of the assets and contracts mentioned in this Clause 2.8.

## **2.9. Other transfer requirements**

If and insofar the transfer of any existing Business Assets or Business Liabilities requires any other acts for the transfer of the legal title to such Business Assets or Business Liabilities than mentioned in this Deed of Assignment, Parties shall satisfy such requirements and comply with the relevant statutory provisions, as soon as reasonably and practically possible.

# **3. TRANSFER OF SELLER LOANS**

## **3.1. Agreement to transfer**

- 3.1.1. Subject to the terms and conditions of this Deed of Assignment the Transferor hereby contributes to
- (A) AMT BV and AMT BV hereby accept from the Transferor, the legal title and economic ownership of the AMT BV Seller Loan; and
  - (B) AMT IP BV and AMT IP BV hereby accept from the Transferor, the legal title and economic ownership of the AMT IP BV Seller Loan.
- 3.1.2. The AMT BV Seller Loan and the AMT IP BV Seller Loan are with the effect as of the signing date of this Deed of Assignment transferred to AMT BV and AMT IP BV, respectively, by way of a contribution in kind (*inbreng in natura*) on the ordinary shares in the capital of AMT BV and AMT IP BV held by the Transferor, without the issuance of new shares. The value of the AMT BV Seller Loan and the AMT IP BV Seller Loan will be recorded as share premium (*agiostorting*) in the books of AMT BV and AMT IP BV.
- 3.1.3. As a result of the contribution set forth in Clause 3.1 above:
- (A) AMT BV is both the debtor and the creditor under the AMT BV Seller Loan; and
  - (B) AMT IP BV is both the debtor and the creditor under the AMT IP BV Seller Loan,
- resulting in the cancellation of the AMT BV Seller Loan and the AMT IP BV Seller Loan.

## **3.2. VAT**

The Transferor and the Transferees are included in a fiscal unity for Dutch VAT purposes within the meaning of article 7 paragraph 4 Dutch VAT Act 1968 (*Wet op de omzetbelasting 1968*) and the Transferor and the Transferees expressly agree that the transfer of the legal title and economic ownership of the Seller Loans occurs within the fiscal unity for Dutch VAT and that therefore no VAT should become due as a result of such transfer.

#### 4. ECONOMIC OWNERSHIP TRANSFER AGREEMENT

The Economic Ownership Transfer Agreement constitutes an integral part of this Deed of Assignment and is hereby incorporated into this Deed of Assignment by this reference. This Deed of Assignment shall in no way impair or affect the Economic Ownership Transfer Agreement that shall remain in full force and effect despite Completion.

#### 5. GOVERNING LAW AND JURISDICTION

##### 5.1. Governing law

This Deed of Assignment is governed by the laws of the Netherlands, but excluding the Vienna Convention for the International Sale of Goods.

##### 5.2. Jurisdiction

The competent court in Amsterdam, the Netherlands, shall have exclusive jurisdiction to settle any dispute in connection with this Deed of Assignment without prejudice to the right of appeal and that of appeal to the Supreme Court.

THUS AGREED AND SIGNED ON 5 APRIL 2012,

**Amsterdam Molecular Therapeutics (AMT) B.V.**

**Amsterdam Molecular Therapeutics (AMT) IP B.V.**

\_\_\_\_\_  
By: /s/Joern Aldag

Title: CEO

\_\_\_\_\_  
By: /s/Joern Aldag

Title: CEO

**Amsterdam Molecular Therapeutics (AMT) Holding N.V.**

\_\_\_\_\_  
By: /s/Joern Aldag

Title: CEO

DATED 16 February 2012

**Amsterdam Molecular Therapeutics (AMT) B.V.**

and

**Amsterdam Molecular Therapeutics (AMT) IP B.V.**

as the Purchasers

**Amsterdam Molecular Therapeutics (AMT) Holding N.V.**

as the Seller

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**AGREEMENT FOR TRANSFER  
OF CERTAIN ASSETS AND  
LIABILITIES OF AMSTERDAM  
MOLECULAR THERAPEUTICS  
(AMT) HOLDING N.V.**

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SCHEDULE 1 (DEFINITIONS)

SCHEDULE 2 (BUSINESS INTELLECTUAL PROPERTY RIGHTS & FURTHER ASSETS AND LIABILITIES)

**THIS AGREEMENT** is made on 16 February 2012

**BETWEEN:**

**Amsterdam Molecular Therapeutics (AMT) B.V.**, a company incorporated under the laws of the Netherlands with its corporate seat in Amsterdam, the Netherlands, registered with the trade register of the Chamber of Commerce with number 34275365;



**Amsterdam Molecular Therapeutics (AMT) IP B.V.**, a company incorporated under the laws of the Netherlands with its corporate seat in Amsterdam, the Netherlands, registered with the trade register of the Chamber of Commerce with number 34275369;

**(Amsterdam Molecular Therapeutics (AMT) B.V. and Amsterdam Molecular Therapeutics (AMT) B.V IP B.V.** collectively also the “**Purchasers**” and each individually a “**Purchaser**”) and

**Amsterdam Molecular Therapeutics (AMT) Holding N.V.**, a company incorporated under the laws of the Netherlands with its corporate seat in Amsterdam, the Netherlands, registered with the trade register of the Chamber of Commerce with number 3330132 (the “**Seller**”)

The parties to this Agreement are hereinafter collectively referred to as the “**Parties**” and individually as a “**Party**”.

**RECITALS:**

- (1) The Seller is involved in the business of the development of human gene based therapies. In connection therewith, the Seller acts as the holding company for the Purchasers and is listed on NYSE Euronext in Amsterdam.
- (2) The Seller and the Purchasers have reached an agreement with respect to the sale and acquisition, as a going concern, of the economic ownership (*economische eigendom*) of the Business (as defined herein), subject to and upon the terms and conditions of this Agreement.
- (3) In connection with the transfer of the economic ownership of the Business, the Seller and the Purchasers have agreed to the transfer by the Seller to the Purchasers of the Seller Loans after signing of the BAA, but prior to the Completion Date unless such transfer has materially adverse consequences for the Seller or the Purchasers.
- (4) The Seller has obtained all internal corporate approvals required for the execution and performance of this Agreement.

**IT IS AGREED** as follows:

**1. INTERPRETATION**

- 1.1. In this Agreement the definitions in Schedule 1 (Definitions) are used.
- 1.2. In this Agreement, unless otherwise specified:
  - 1.2.1. the masculine gender shall include the feminine and the neuter and vice versa;
  - 1.2.2. references to a person shall include a reference to any individual, company, association, partnership or joint venture;
  - 1.2.3. references to “include” and “including” shall be treated as references to “include without limitation” or “including without limitation”;
  - 1.2.4. references to documents in “agreed form” shall be to documents agreed between the Parties, annexed to this Agreement and initialled for identification by the Parties;
  - 1.2.5. unless the context requires otherwise, words in the singular shall include the plural and vice versa;
  - 1.2.6. the headings are for identification only and shall not affect the interpretation of this Agreement.

**2. TRANSFER**

- 2.1. **Sale of the Business**
  - 2.1.1. Subject to the terms and conditions of this Agreement the Seller hereby contributes to the Purchasers and the Purchasers hereby accept or assume (as the case may be), as a going concern, from the Seller, the economic ownership of the Business with the effect as of the signing date of this Agreement (the **Effective Date**), whereby:
    - (A) the economic ownership of the Business Intellectual Property Rights is hereby transferred to AMT IP BV; and
    - (B) the economic ownership of the other Business Assets and Business Liabilities are hereby transferred to AMT BV.
  - 2.1.2. The Seller expressly confirms that the Purchasers shall not purchase or assume any other assets and liabilities than the Business Assets and Business Liabilities, and that all other assets and liabilities are excluded from the sale and purchase of the Business, including the Excluded Contracts.
  - 2.1.3. The economic benefit and burden of the Business shall be for the risk and account of the Purchasers with effect as of the Effective Date, it being understood that the Business Assets and Business Liabilities, to the extent they relate to any period after the Effective Date, shall be for the risk and account of the Purchasers and that the Business Assets and Business Liabilities, to the extent they relate to any period before the Effective Date, shall be for the risk and account of the Seller.
  - 2.1.4. The Seller covenants that it has the right to sell and transfer or assign (as the case may be) to the Purchasers the economic ownership of the Business on the terms and conditions set out in this Agreement.
  - 2.1.5. In connection with the contribution set forth in Clause 2.1.1 above, the Seller hereby undertakes to transfer the legal title (*juridische eigendom*) to the Business to the Purchasers in accordance with Clause 3, whereby:
    - (A) the legal title to the Business Intellectual Property Rights will be transferred to AMT IP BV; and

transferred to AMT BV.

- 2.1.6. The respective parts of the Business shall be transferred to AMT IP BV and AMT BV by way of a contribution in kind (*inbreng in natura*) on the ordinary shares in the capital of the Purchasers held by the Seller, without the issuance of new shares. The value of the respective parts of the Business will be recorded as share premium (*agiostorting*) in the books of AMT IP BV and AMT BV.

## 2.2. Sale of the Seller Loans

The Seller and the Purchasers hereby agree to the transfer by the Seller to the Purchasers of the Seller Loans after signing of the BAA, but prior to the Completion Date unless such transfer has materially adverse consequences for the Seller or the Purchasers; such determination to be made by the Seller and the Purchasers jointly.

## 2.3. VAT

The Seller and the Purchasers are included in a fiscal unity for Dutch VAT purposes within the meaning of article 7 paragraph 4 Dutch VAT Act 1968 (*Wet op de omzetbelasting 1968*) and the Seller and the Purchasers expressly agree that the transfer of the economic ownership of the Business occurs within the fiscal unity for Dutch VAT and that therefore no VAT should become due as a result of such transfer.

## 3. TRANSFER OF LEGAL TITLE

The transfer of the legal title to the Business by the Seller to the Purchasers, as meant in Clause 2.1.5, shall be implemented by means of the execution of a deed of assignment (the “Deed of Assignment”) and in connection therewith the Seller shall undertake all necessary actions, including but not limited to:

- (A) informing the debtors of the Accounts Receivable in writing that the Accounts Receivable have been assigned to the Purchaser);
- (B) requesting the counterparties to the Contracts in writing for their co-operation to the transfer of contract) to which the Seller is a party; and
- (C) to the extent not already referred to in this Clause 3, the proper fulfilment of the applicable transfer requirements in respect of the Further Assets and Liabilities owned and/or held by the Seller.

The Parties shall take any and all further actions as needed to transfer the legal title to the Business from the Seller to the Purchasers.

## 4. CONFIDENTIALITY AND ANNOUNCEMENTS

### 4.1. Confidentiality

- 4.1.1. Subject to Clause 4.1.2 and Clause 4.2, each Party shall treat as strictly confidential and not disclose or use any information relating to this Agreement or any ancillary matter and the negotiations leading up to this Agreement. The Seller shall not disclose or use any information in its possession relating to the Business following Completion and any information relating to the Purchaser.

- 4.1.2. The restrictions contained in Clause 4.1.1 shall not apply if and to the extent:

- (A) disclosure is required by any Law or by a court;
- (B) disclosure is required by any securities exchange or regulatory or governmental body;
- (C) disclosure is necessary to enforce this Agreement in court proceedings.
- (D) the other Party has given its written consent to disclosure;
- (E) the information has come into the public domain through no fault of the relevant Party’s group;
- (F) disclosure is necessary to obtain the advice of any professional adviser.

In the event of a disclosure of information pursuant to Clause 4.1.2 (A) or (B), the disclosing Party shall consult with the other Party as to the contents, form and timing of the disclosure to be made.

- 4.1.3. Each of the Parties shall ensure that each of its shareholder(s), subsidiaries, participations, managing directors or other employees are bound by and observe the restrictions in Clause 4.1.

### 4.2. Announcements

- 4.2.1. Subject to Clause 4.2.1, none of the Parties shall make any announcement before or after Completion with respect to this Agreement or any ancillary matter without the prior written consent of the other Party, that consent not to be unreasonably withheld or delayed.
- 4.2.2. Notwithstanding Clause 4.2.1, a Party may make an announcement with respect to this Agreement or any ancillary matter if required by any Law to which that Party is subject, provided that any such announcement shall be made by such Party only after consultation with the other

Party.

## 5. GENERAL PROVISIONS

### 5.1. Notices

All announcements or notices to the Parties will be done in writing and delivered to the relevant Party at its address specified in Schedule 8 of the BAA as long as a Party does not give notice to the other Parties of any other address.

### 5.2. Entire agreement

This Agreement constitutes the entire agreement between the Parties relating to the transfer of the economic ownership of the Business. This Agreement supersedes and terminates any preceding or concurrent oral or written agreements between the Parties and no Party shall have any right or remedy against any other Party arising out of or in connection with any such preceding or concurrent agreements unless stated otherwise in this Agreement.

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### 5.3. Amendment

This Agreement may only be amended by mutual agreement in writing.

### 5.4. Assignment

None of the Parties may assign or procure the assumption of its rights and obligations under this Agreement, either in whole or in part, to any other person without the prior written consent of the other Party.

### 5.5. Partial invalidity

The invalidity or unenforceability of any provision of this Agreement shall not affect the validity or enforceability of any other provision of this Agreement. Any such invalid or unenforceable provision shall be replaced or be deemed to be replaced by a provision that is considered to be valid and enforceable. The interpretation of the replacing provisions shall be as close as possible to the intent of the invalid or unenforceable provision.

### 5.6. Rescission after Completion

The Parties waive their right to rescind (*ontbinden*) this Agreement pursuant to Article 6:265 of the Dutch Civil Code after Completion.

### 5.7. Governing law

This Agreement is governed by the laws of the Netherlands, but excluding the Vienna Convention for the International Sale of Goods.

### 5.8. Jurisdiction

The competent court in Amsterdam, the Netherlands shall have exclusive jurisdiction to settle any dispute in connection with this Agreement without prejudice to the right of appeal and that of appeal to the Supreme Court.

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THUS AGREED AND SIGNED IN AMSTERDAM ON 16 FEBRUARY 2012,

**Amsterdam Molecular Therapeutics (AMT) B.V.**

**Amsterdam Molecular Therapeutics (AMT) IP B.V.**

By: /s/Joern Aldag, CEO

By: /s/Joern Aldag, CEO

By: /s/Piers Morgan, CFO

By: /s/Piers Morgan, CFO

**Amsterdam Molecular Therapeutics (AMT) Holding N.V.**

By: /s/Joern Aldag, CEO

/s/Piers Morgan, CFO

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## SCHEDULE 1 (DEFINITIONS)

“**Accounts Receivable**” means all the accounts receivable of the Seller relating to the Business;

“**Administration**” means all the administration of the Group relating to the Business, whether in electronic or physical form, including but not limited to ownership titles of assets, bought and sold ledgers, purchase and sales day books and purchase and sales invoices, management information records and other accounting books and records of the Group including tax records relating to the Business;

“**Agreement**” means this Agreement for the sale and purchase of the economic ownership of certain assets and liabilities of the Seller to the Purchasers including the recitals and all Schedules, as amended in accordance with its terms;

“**AMT BV**” means Amsterdam Molecular Therapeutics (AMT) B.V.;

“**AMT BV Seller Loan**” means the intra Group loan agreement between the Seller (as lender) and AMT BV (as borrower) and all (existing and future) rights and obligations there under;

“**AMT BV Shares**” means all of the issued and outstanding shares in the share capital of AMT BV;

“**AMT IP BV**” means Amsterdam Molecular Therapeutics (AMT) IP B.V.;

“**AMT IP BV Seller Loan**” means the intra Group loan agreement between the Seller (as lender) and AMT IP BV (as borrower) and all (existing and future) rights and obligations thereunder;

“**AMT IP BV Shares**” means all of the issued and outstanding shares in the share capital of AMT IP BV;

“**BAA**” means the Business Acquisition Agreement entered on 16 February 2012 between the Seller, Kairos Therapeutics B.V. as the purchaser, Forbion Co-Investment II Coöperatief U.A., Coöperatieve AAC LS U.A. and FORBION Co-Investment COÖPERATIEF U.A. as the Investor, AMT BV and AMT IP BV as the Subsidiaries.

“**Business**” means all activities of the Seller, its operations and the Business Assets and the Business Liabilities;

“**Business Assets**” means all assets of the Sellers, including but not limited to its rights in respect of the Contracts, the Business Intellectual Property Rights, the Further Assets and Liabilities, the Business Goodwill and Know How, and any other asset related to the Business, and that have not expressly been excluded and excluding the Sale Shares and the Seller Loans;

“**Business Goodwill and Know How**” means any and all goodwill with respect to the Business, irrespective of whether such goodwill has been capitalised (*geactiveerd*), and all technical and other information in any form which is not in the public domain in respect of the Business (other than Business Intellectual Property Rights);

“**Business Intellectual Property Rights**” means the intellectual property rights of the Seller in relation to the Business, such as but not limited to (a) patents, trade marks, service marks, registered designs, trade, business and company names, internet domain names and e-mail addresses, unregistered trade marks and service marks, copyrights, database rights, know how,

rights in designs and inventions and applications and rights to apply for any of those rights; (b) the rights to sue for past infringements of any of the foregoing rights, including the intellectual property rights listed in Schedule 2;

“**Business Liabilities**” means all bona fide liabilities of the Seller in respect of the Business that have not expressly been excluded, and all other liabilities expressly assumed by the Purchasers under this Agreement, excluding the Loan Notes and the Convertible Loan Note Agreement;

“**Business Day**” means a day, other than a Saturday or a Sunday, on which the banks in Amsterdam are open for normal business;

“**Completion Date**” means the date on which the completion of the BAA shall occur;

“**Contracts**” means all contracts entered into by the Seller relating to the Business and all (existing and future) rights and obligations thereunder, including those specified in Schedule 2, but excluding the Excluded Contracts;

“**Convertible Loan Note Agreement**” means the agreement constituting the issuance of loan notes dated 22 December 2009 and made by the Seller and Coöperatieve AAC LS U.A. and Forbion Co-Investment Coöperatief U.A. and all existing and future rights and obligations of the Seller there under;

“**Excluded Contracts**” means this Agreement, the BAA, the listing agreement between the Seller and Euronext Amsterdam, the contract with the ENL agent and the payment agent, the liquidity provider agreement between the Seller and Kempen & Co N.V., the D&O Insurance policy of the Seller and any outstanding obligations under any stock option plan or other employee benefit plan of the Seller;

“**Further Assets and Liabilities**” means the Accounts Receivable, Intra-Group Trading Items and Contracts and any further assets and liabilities of the Group as further specified in Schedule 2;

“**Group**” means the Seller and the Purchasers collectively;

“**Intra-Group Trading Items**” means at any time, and from time to time, all amounts owed, outstanding or accrued in the ordinary course of trading as between the Seller and the Purchasers in respect of intra group trading activity between them;

“**Law**” means any law, regulation, directive, covenant, guideline, standard, circular or general policy rule of any governmental or regulatory body in any jurisdiction;

“**Loan Note 1**” means the €700,000 5% convertible loan note due 2014 of the Seller dated 23 December 2009 and issued to and held by Coöperatieve AAC LS U.A. which is constituted by the Convertible Loan Note Agreement, and all the existing and future rights and obligations of the Seller there under;

“**Loan Note 2**” means the €4,300,000 5% convertible loan note due 2014 of the Seller dated 23 December 2009 and issued to and held by Forbion Co-Investment Coöperatief U.A. which is constituted by the Convertible Loan Note Agreement, and all the existing and future rights and obligations of the Seller there under;

“**Loan Notes**” means the Loan Note 1 and the Loan Note 2 collectively;

“**Purchaser**” has the meaning given in the opening of this Agreement;

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“**Reorganisation**” has the meaning given in recital (E) of the BAA;

“**Sale Shares**” means the AMT IP BV Shares and the AMT BV Shares;

“**Seller**” has the meaning given in the opening of this Agreement;

“**Seller Loans**” means the AMT BV Seller Loan and the AMT IP BV Seller Loan collectively;

“**Signing Date**” means 16 February 2012, being the date on which the BAA is signed and dated;






“**VAT**” means value added taxation within the meaning of the VAT Act 1968 (*Wet op de omzetbelasting 1968*).

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## SCHEDULE 2

### (BUSINESS INTELLECTUAL PROPERTY RIGHTS & FURTHER ASSETS AND LIABILITIES)

#### LIST OF TRADEMARKS

	Catchword	Type	Country	Classes	Appl.No.	Appl.date	Reg.No.	Reg.date	Ren.date	Applicant	Status	Case No.	Watch
1.		Logotype	IL	05	209906	24-03-08.	209906	07-02-10.	24-03-18.	Amsterdam Molecular Therapeutics (AMT) Holding N.V.	Registered	T56005IL00	No
2.		Logotype	IS	05	10692009	22-04-09.	3772009	02-06-09.	02-06-19.	Amsterdam Molecular Therapeutics (AMT) Holding N.V.	Registered	T56005IS00	No
3.		Logotype	JO	05	100494	23-04-08.	100494	23-04-08.	23-04-18.	Amsterdam Molecular Therapeutics (AMT) Holding N.V.	Registered	T56005JO00	No
4.		Logotype	NO	05	200905089	21-04-09.	251774	14-07-09.	14-07-19.	Amsterdam Molecular Therapeutics (AMT) Holding N.V.	Registered	T56005NO00	No
5.		Logotype	TR	05	200925333	18-05-09.	200925333	04-05-10.	18-05-19.	Amsterdam Molecular Therapeutics (AMT) Holding N.V.	Registered	T56005TR00	No
6.	DELIVERING CURE	Wordmark	AE	05	113972	03-06-08.	150481	09-12-11.	03-06-18.	Amsterdam Molecular Therapeutics (AMT) Holding N.V.	Registered	T56004AE00	No
7.	DELIVERING CURE	Wordmark	BH	05	64727	18-03-08.				Amsterdam Molecular Therapeutics (AMT) Holding N.V.	Pending	T56004BH00	No
8.	DELIVERING CURE	Wordmark	IR	05	86122678	18-03-08.	157479	14-09-08.	18-03-18.	Amsterdam Molecular Therapeutics (AMT) Holding N.V.	Registered	T56004IR00	No
9.	DELIVERING CURE	Wordmark	LB	05	2449	08-04-08.	116062	24-04-08.	24-04-23.	Amsterdam Molecular Therapeutics (AMT) Holding N.V.	Registered	T56004LB00	No
10.	DELIVERING CURE	Wordmark	LY	05	17093	05-02-09.				Amsterdam Molecular Therapeutics (AMT) Holding N.V.	Pending	T56004LY00	No
11.	DELIVERING CURE	Wordmark	MA	05	118083	19-06-08.	118083	17-11-08.	19-06-18.	Amsterdam Molecular Therapeutics (AMT) Holding N.V.	Registered	T56004MA00	No

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12.	DELIVERING CURE	Wordmark	OM	05	49398	19-03-08.	49398	11-08-09.	19-03-18.	Amsterdam Molecular Therapeutics (AMT) Holding N.V.	Registered	T56004OM00	No
13.	DELIVERING CURE	Wordmark	QA	05	50165	03-04-08.	50165	20-03-11.	03-04-18.	Amsterdam Molecular Therapeutics (AMT) Holding N.V.	Registered	T56004QA00	No
14.	DELIVERING CURE	Wordmark	RU	05	2008707490	14-03-08.	381651	16-06-09.	14-03-18.	Amsterdam Molecular Therapeutics (AMT) Holding N.V.	Registered	T56004RU00	No
15.	DELIVERING CURE	Wordmark	SY	05	3814	22-04-08.				Amsterdam Molecular Therapeutics (AMT) Holding N.V.	Pending	T56004SY00	No
16.	DELIVERING CURE	Wordmark	TN	05	EE080755	19-03-08.	EE080755	26-01-10.	19-03-18.	Amsterdam Molecular Therapeutics (AMT) Holding N.V.	Registered	T56004TN00	No
17.	DELIVERING CURE	Wordmark	US	05	77/421590	13-03-08.				Amsterdam Molecular Therapeutics (AMT) Holding N.V.	Pending	T56004US00	No
18.	DELIVERING CURE	Wordmark	ZA	05	200805836	14-03-08.	200805836	14-03-08.	14-03-18.	Amsterdam Molecular Therapeutics (AMT) Holding N.V.	Registered	T56004ZA00	No
19.	GLYBERA	Wordmark	AE	05	101941	31-10-07.				Amsterdam Molecular Therapeutics (AMT) Holding N.V.	Pending	T56001AE00	Yes

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20.	GLYBERA	Wordmark	AU	05	1176048	14-05-07.	1176048	12-12-07.	14-05-17.	Amsterdam Molecular Therapeutics (AMT) Holding N.V.	Registered	T56001AU00	Yes
21.	GLYBERA	Wordmark	BH	05	62689	07-01-08.	62689	07-01-08.	07-01-18.	Amsterdam Molecular Therapeutics (AMT) Holding N.V.	Registered	T56001BH00	Yes
22.	GLYBERA	Wordmark	DZ	05	72791	24-10-07.				Amsterdam Molecular Therapeutics (AMT) Holding N.V.	Pending	T56001DZ00	Yes
23.	GLYBERA	Wordmark	EG	05	208229	22-10-07.				Amsterdam Molecular Therapeutics (AMT) Holding N.V.	Pending	T56001EG00	Yes
24.	GLYBERA	Wordmark	IL	05	204800	21-10-07.	204800	11-08-09.	21-10-17.	Amsterdam Molecular Therapeutics (AMT) Holding N.V.	Registered	T56001IL00	Yes
25.	GLYBERA	Wordmark	IS	05	14642007	14-05-07.	8122007	04-07-07.	04-07-17.	Amsterdam Molecular Therapeutics (AMT) Holding N.V.	Registered	T56001IS00	Yes
26.	GLYBERA	Wordmark	JO	05	99133	24-10-07.	99133	01-05-07.	01-05-17.	Amsterdam Molecular Therapeutics (AMT) Holding N.V.	Registered	T56001JO00	Yes
27.	GLYBERA	Wordmark	LB	05	6612	23-10-07.	113370	25-10-07.	25-10-22.	Amsterdam Molecular Therapeutics (AMT) Holding N.V.	Registered	T56001LB00	Yes
28.	GLYBERA	Wordmark	LY	05	16593	22-12-08.				Amsterdam Molecular Therapeutics (AMT) Holding N.V.	Pending	T56001LY00	Yes

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29.	GLYBERA	Wordmark	MA	05	113550	23-10-07.	113550	23-10-07.	23-10-17.	Amsterdam Molecular Therapeutics (AMT) Holding N.V.	Registered	T56001MA00	Yes
30.	GLYBERA	Wordmark	NO	05	200705606	15-05-07.	241553	19-10-07.	19-10-17.	Amsterdam Molecular Therapeutics (AMT) Holding N.V.	Registered	T56001NO00	Yes
31.	GLYBERA	Wordmark	NZ	05	768310	14-05-07.	768310	15-11-07.	14-05-17.	Amsterdam Molecular Therapeutics (AMT) Holding N.V.	Registered	T56001NZ00	Yes
32.	GLYBERA	Wordmark	OM	05	47462	22-10-07.	47462	24-08-08.	22-10-17.	Amsterdam Molecular Therapeutics (AMT) Holding N.V.	Registered	T56001OM00	Yes
33.	GLYBERA	Wordmark	QA	05	47253	31-10-07.	47253	31-12-09.	31-10-17.	Amsterdam Molecular Therapeutics (AMT) Holding N.V.	Registered	T56001QA00	Yes
34.	GLYBERA	Wordmark	RU	05	2008707340	13-03-08.	377215	20-04-09.	13-03-18.	Amsterdam Molecular Therapeutics (AMT) Holding N.V.	Registered	T56001RU00	Yes
35.	GLYBERA	Wordmark	SA	05	125692	12-01-	1156/45	25-04-	12-09-	Amsterdam Molecular	Registered	T56001SA00	Yes

						08.		10.	17.	Therapeutics (AMT) Holding N.V.			
36.	GLYBERA	Wordmark	SY	05	4268	28-10-07.				Amsterdam Molecular Therapeutics (AMT) Holding N.V.	Pending	T56001SY00	Yes
37.	GLYBERA	Wordmark	TN	05	EE072667	24-10-07.	EE072667	19-05-09.	24-10-17.	Amsterdam Molecular Therapeutics (AMT) Holding N.V.	Registered	T56001TN00	Yes

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38.	GLYBERA	Wordmark	TR	05	2007026778	17-05-07.	200726778	17-05-07.	17-05-17.	Amsterdam Molecular Therapeutics (AMT) Holding N.V.	Registered	T56001TR00	Yes
39.	GLYBERA	Wordmark	ZA	05	200723919	19-10-07.	2007/23919	19-10-07.	19-10-17.	Amsterdam Molecular Therapeutics (AMT) Holding N.V.	Registered	T56001ZA00	Yes
40.	VECTIPRO	Wordmark	AE	05	101942	31-10-07.	100927	24-03-10.	31-10-17.	Amsterdam Molecular Therapeutics (AMT) Holding N.V.	Registered	T56002AE00	Yes
41.	VECTIPRO	Wordmark	AU	05	1176051	14-05-07.	1176051	12-12-07.	14-05-17.	Amsterdam Molecular Therapeutics (AMT) Holding N.V.	Registered	T56002AU00	Yes
42.	VECTIPRO	Wordmark	BH	05	62690	07-01-08.	62690	07-01-08.	07-01-18.	Amsterdam Molecular Therapeutics (AMT) Holding N.V.	Registered	T56002BH00	Yes
43.	VECTIPRO	Wordmark	DZ	05	72793	24-10-07.				Amsterdam Molecular Therapeutics (AMT) Holding N.V.	Pending	T56002DZ00	Yes
44.	VECTIPRO	Wordmark	EG	05	208203	22-10-07.				Amsterdam Molecular Therapeutics (AMT) Holding N.V.	Pending	T56002EG00	Yes
45.	VECTIPRO	Wordmark	IL	05	204915	23-10-07.	204915	11-08-09.	23-10-17.	Amsterdam Molecular Therapeutics (AMT) Holding N.V.	Registered	T56002IL00	Yes
46.	VECTIPRO	Wordmark	IR	05	86091403	08-12-07.	157475	14-09-08.	08-12-17.	Amsterdam Molecular Therapeutics (AMT) Holding N.V.	Registered	T56002IR00	Yes

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47.	VECTIPRO	Wordmark	IS	05	14632007	14-05-07.	8112007	04-07-07.	04-07-17.	Amsterdam Molecular Therapeutics (AMT) Holding N.V.	Registered	T56002IS00	Yes
48.	VECTIPRO	Wordmark	JO	05	99366	24-10-07.	99366	14-01-09.	01-05-17.	Amsterdam Molecular Therapeutics (AMT) Holding N.V.	Registered	T56002JO00	Yes
49.	VECTIPRO	Wordmark	LB	05	6622	23-10-07.	113434	30-10-07.	30-10-22.	Amsterdam Molecular Therapeutics (AMT) Holding N.V.	Registered	T56002LB00	Yes
50.	VECTIPRO	Wordmark	LY	05	16595	22-12-08.				Amsterdam Molecular Therapeutics (AMT) Holding N.V.	Pending	T56002LY00	Yes
51.	VECTIPRO	Wordmark	MA	05	113551	23-10-07.	113551	23-10-07.	23-10-17.	Amsterdam Molecular Therapeutics (AMT) Holding N.V.	Registered	T56002MA00	Yes
52.	VECTIPRO	Wordmark	NO	05	200705604	15-05-07.	241558	22-10-07.	22-10-17.	Amsterdam Molecular Therapeutics (AMT) Holding N.V.	Registered	T56002NO00	Yes
53.	VECTIPRO	Wordmark	NZ	05	768309	14-05-07.	768309	12-02-09.	01-05-17.	Amsterdam Molecular Therapeutics (AMT) Holding N.V.	Registered	T56002NZ00	Yes
54.	VECTIPRO	Wordmark	OM	05	47461	22-10-07.	47461	30-05-09.	22-10-17.	Amsterdam Molecular Therapeutics (AMT) Holding N.V.	Registered	T56002OM00	Yes
55.	VECTIPRO	Wordmark	QA	05	47255	31-10-07.	47255	31-12-09.	31-10-17.	Amsterdam Molecular Therapeutics (AMT) Holding N.V.	Pending	T56002QA00	Yes

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56.	VECTIPRO	Wordmark	RU	05	2008707342	13-03-08.	381400	10-06-09.	13-03-18.	Amsterdam Molecular Therapeutics (AMT) Holding N.V.	Registered	T56002RU00	Yes
57.	VECTIPRO	Wordmark	SA	05	125693	12-01-	1171/48	19-06-	12-09-	Amsterdam Molecular	Registered	T56002SA00	Yes

					08.		10.	17.	Therapeutics (AMT) Holding N.V.				
58.	VECTIPRO	Wordmark	SY	05	4269	28-10-07.			Amsterdam Molecular Therapeutics (AMT) Holding N.V.	Pending	T56002SY00	Yes	
59.	VECTIPRO	Wordmark	TN	05	EE072666	24-10-07.	EE072666	19-05-09.	24-10-17.	Amsterdam Molecular Therapeutics (AMT) Holding N.V.	Registered	T56002TN00	Yes
60.	VECTIPRO	Wordmark	TR	05	2007026779	17-05-07.	200726779	17-05-07.	17-05-17.	Amsterdam Molecular Therapeutics (AMT) Holding N.V.	Registered	T56002TR00	Yes
61.	VECTIPRO	Wordmark	ZA	05	200723918	19-10-07.	200723918	19-10-07.	19-10-17.	Amsterdam Molecular Therapeutics (AMT) Holding N.V.	Registered	T56002ZA00	Yes
62.	ZYAMTIN	Wordmark	AE	05	101943	31-10-07.	100909	24-03-10.	31-10-17.	Amsterdam Molecular Therapeutics (AMT) Holding N.V.	Registered	T56003AE00	Yes
63.	ZYAMTIN	Wordmark	AU	05	1176049	14-05-07.	1176049	12-12-07.	14-05-17.	Amsterdam Molecular Therapeutics (AMT) Holding N.V.	Registered	T56003AU00	Yes
64.	ZYAMTIN	Wordmark	BH	05	62691	07-01-08.	62691	07-01-08.	07-01-18.	Amsterdam Molecular Therapeutics (AMT) Holding N.V.	Registered	T56003BH00	Yes

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65.	ZYAMTIN	Wordmark	DZ	05	72792	24-10-07.				Amsterdam Molecular Therapeutics (AMT) Holding N.V.	Pending	T56003DZ00	Yes
66.	ZYAMTIN	Wordmark	EG	05	208231	22-10-07.				Amsterdam Molecular Therapeutics (AMT) Holding N.V.	Pending	T56003EG00	Yes
67.	ZYAMTIN	Wordmark	IL	05	204799	21-10-07.	204799	11-04-09.	21-10-17.	Amsterdam Molecular Therapeutics (AMT) Holding N.V.	Registered	T56003IL00	Yes
68.	ZYAMTIN	Wordmark	IR	05	86091401	08-12-07.	158201	14-09-08.	08-12-17.	Amsterdam Molecular Therapeutics (AMT) Holding N.V.	Registered	T56003IR00	Yes
69.	ZYAMTIN	Wordmark	IS	05	14652007	14-05-07.	8132007	04-07-07.	04-07-17.	Amsterdam Molecular Therapeutics (AMT) Holding N.V.	Registered	T56003IS00	Yes
70.	ZYAMTIN	Wordmark	JO	05	99208	24-10-07.	99208	03-03-09.	01-05-17.	Amsterdam Molecular Therapeutics (AMT) Holding N.V.	Registered	T56003JO00	Yes
71.	ZYAMTIN	Wordmark	LB	05	6623	23-10-07.	113437	30-10-07.	30-10-22.	Amsterdam Molecular Therapeutics (AMT) Holding N.V.	Registered	T56003LB00	Yes
72.	ZYAMTIN	Wordmark	LY	05	16594	22-12-08.				Amsterdam Molecular Therapeutics (AMT) Holding N.V.	Pending	T56003LY00	Yes
73.	ZYAMTIN	Wordmark	MA	05	113552	23-10-07.	113552	23-10-07.	23-10-17.	Amsterdam Molecular Therapeutics (AMT) Holding N.V.	Registered	T56003MA00	Yes

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74.	ZYAMTIN	Wordmark	NO	05	200705605	15-05-07.	241517	18-10-07.	18-10-17.	Amsterdam Molecular Therapeutics (AMT) Holding N.V.	Registered	T56003NO00	Yes
75.	ZYAMTIN	Wordmark	NZ	05	768311	14-05-07.	768311	15-11-07.	14-05-17.	Amsterdam Molecular Therapeutics (AMT) Holding N.V.	Registered	T56003NZ00	Yes
76.	ZYAMTIN	Wordmark	OM	05	47463	22-10-07.	47463	30-05-09.	22-10-17.	Amsterdam Molecular Therapeutics (AMT) Holding N.V.	Registered	T56003OM00	Yes
77.	ZYAMTIN	Wordmark	QA	05	47254	31-10-07.	47254	31-12-09.	31-10-17.	Amsterdam Molecular Therapeutics (AMT) Holding N.V.	Registered	T56003QA00	Yes
78.	ZYAMTIN	Wordmark	RU	05	2008707341	13-03-08.	394999	01-12-09.	13-03-18.	Amsterdam Molecular Therapeutics (AMT) Holding N.V.	Registered	T56003RU00	Yes
79.	ZYAMTIN	Wordmark	SA	05	125694	12-01-08.	1171/49	19-06-10.	12-09-17.	Amsterdam Molecular Therapeutics (AMT) Holding N.V.	Registered	T56003SA00	Yes
80.	ZYAMTIN	Wordmark	SY	05	4267	28-10-07.				Amsterdam Molecular Therapeutics (AMT) Holding N.V.	Pending	T56003SY00	Yes
81.	ZYAMTIN	Wordmark	TN	05	EE072668	24-10-07.	EE072668	19-05-09.	24-10-17.	Amsterdam Molecular Therapeutics (AMT) Holding N.V.	Registered	T56003TN00	Yes
82.	ZYAMTIN	Wordmark	TR	05	2007026780	17-05-07.	200726780	07-04-08.	17-05-17.	Amsterdam Molecular Therapeutics (AMT) Holding N.V.	Registered	T56003TR00	Yes

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83.	ZYAMTIN	Wordmark	US	05	77/179359	11-05-07.	3855311	05-10-10.	05-10-20.		Registered	T56003US00	Yes
84.	ZYAMTIN	Wordmark	ZA	05	200723917	19-10-07.	200723917	14-07-10.	19-10-17.	Amsterdam Molecular Therapeutics (AMT) Holding N.V.	Registered	T56003ZA00	Yes

SUBSIDIARIES OF THE REGISTRANT

Name of Subsidiary	Jurisdiction of incorporation or organization
uniQure biopharma B.V.	The Netherlands
uniQure IP B.V.	The Netherlands
uniQure Inc.	Delaware
uniQure manufacturing B.V.	The Netherlands
uniQure Assay Development B.V.	The Netherlands
uniQure Research B.V.	The Netherlands
uniQure non clinical B.V.	The Netherlands
uniQure QA B.V.	The Netherlands
uniQure process development B.V.	The Netherlands
uniQure clinical B.V.	The Netherlands

CONSENT OF INDEPENDENT REGISTERED PUBLIC ACCOUNTING FIRM

We hereby consent to the use in this Registration Statement on Form F-1 of uniQure B.V. of our report dated October 25, 2013 relating to the financial statements of uniQure B.V., which appears in such registration statement. We also consent to the reference to us under the heading “Experts” in such Registration Statement.

PricewaterhouseCoopers Accountants N.V.  
Utrecht, The Netherlands  
January 2, 2014

/s/ drs. A.C.M. van der Linden RA

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December 19, 2013

Securities and Exchange Commission  
 Division of Corporation Finance  
 Office of the Chief Accountant  
 100F Street NE  
 Washington, DC 20549

Re: uniQure B.V. Registration Statement on Form F-1 Confidentially Submitted November 8, 2013 and December 19, 2013 - Application for Waiver of Requirements of Form 20-F, Item 8.A.4

Ladies and Gentlemen:

On behalf of our client, uniQure B.V. a Dutch private company with limited liability (*besloten vennootschap met beperkte aansprakelijkheid*) (the “Company”), and in connection with a proposed initial public offering of the Company’s ordinary shares, we hereby respectfully request that the Securities and Exchange Commission (the “Commission”) waive the requirement of Item 8.A.4 of Form 20-F, which states that in the case of a company’s initial public offering (“IPO”) the Registration Statement on Form F-1 (the “Registration Statement”) must contain audited financial statements of a date not older than 12 months from the date of the offering unless a waiver is obtained. *See also* Division of Corporation Finance, *Financial Reporting Manual*, Section 6220.3.

At the time of initial submission on November 8, 2013, the Company’s Registration Statement satisfied Item 8.A.4 of Form 20-F, which is applicable to the Registration Statement pursuant to Item 4(a) of Form F-1, because it contains audited financial statements for the two years ended December 31, 2011 and 2012 and unaudited financial statements for the six months ended June 30, 2012 and 2013, and at the time of our second confidential submission on December 19, 2013, the Company’s Registration Statement satisfied Item 8.A.4 of Form 20-F, because it contains audited financial statements for the two years ended December 31, 2011 and 2012 and unaudited financial statements for the nine months ended September 30, 2012 and 2013, in each case prepared in accordance with International Financial Reporting Standards. However, the Company anticipates filing at least one amendment after December 31, 2013 containing the same financial statements as those that are contained in its second confidential filing because its audited financial statements will not be available until March 2014.

The Company is submitting this waiver request pursuant to Instruction 2 to Item 8.A.4 of Form 20-F, which provides that the Commission will waive the 12-month age of financial statements requirement “in cases where the company is able to represent adequately to us that it is not required to comply with this requirement in any other jurisdiction outside the United States and that complying with this requirement is impracticable or involves undue hardship.”

Wilmer Cutler Pickering Hale and Dorr LLP, Alder Castle, 10 Noble Street, London EC2V 7QJ  
 Beijing Berlin Boston Brussels Frankfurt London Los Angeles New York Oxford Palo Alto Waltham Washington

WilmerHale is the trading name of Wilmer Cutler Pickering Hale and Dorr LLP, a Delaware limited liability partnership of solicitors and registered foreign lawyers authorised and regulated by the Solicitors' Regulation Authority (SRA No. 287489). Our professional rules can be found at [www.sra.org.uk/solicitors/code-of-conduct.page](http://www.sra.org.uk/solicitors/code-of-conduct.page). A list of partners and their professional qualifications is available for inspection at our UK offices. Outside the United Kingdom, Wilmer Cutler Pickering Hale and Dorr LLP operates under a separate Delaware limited liability partnership.

See also the Staff’s 2004 release entitled *International Reporting and Disclosure Issues in the Division of Corporation Finance* (available on the Commission’s website at <http://www.sec.gov/divisions/corpfin/internatl/cfirdissues1104.htm>) at Section III.B.c, in which the Staff notes:

“the instruction indicates that the staff will waive the 12-month requirement where it is not applicable in the registrant’s other filing jurisdictions and is impracticable or involves undue hardship. As a result, we expect that the vast majority of IPOs will be subject only to the 15-month rule. The only times that we anticipate audited financial statements will be filed under the 12-month rule are when the registrants must comply with the rule in another jurisdiction, or when those audited financial statements are otherwise readily available.”

In connection with this request, we as counsel to the Company, represent to the Commission that:

1. The Company is not currently a public reporting company in any other jurisdiction.
2. The Company is not required by any jurisdiction outside the United States to prepare, and has not prepared, financial statements audited under any generally accepted auditing standards for any interim period.
3. Compliance with Item 8.A.4 is impracticable and involves undue hardship for the Company.
4. The Company does not anticipate that its audited financial statements for the year ended December 31, 2013 will be available until March 2014.
5. In no event will the Company seek effectiveness of the Registration Statement if its audited financial statements are older than 15 months at the time of the offering.

We will file this letter as an exhibit to the Registration Statement pursuant to Instruction 2 to Item 8.A.4 of Form 20-F.

Please do not hesitate to contact David Redlick at (617) 526-6434 or Timothy Corbett at +44 20 7645 2509, both of WilmerHale LLP, if you have any questions regarding the foregoing or if we can provide any additional information.

Very truly yours,

/s/ WilmerHale LLP  
 WilmerHale LLP

cc: Piers Morgan, Chief Financial Officer, uniQure B.V.

