As filed with the Securities and Exchange Commission on January 17, 2014

Registration no. 333-193158

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

Amendment No. 1
Form F-1
REGISTRATION STATEMENT
UNDER
THE SECURITIES ACT OF 1933

UNIQURE B.V.*
(Exact Name of Registrant as Specified in Its Charter)

N/A
(Translation of Registrant's Name into English)

The Netherlands	**2834**	**Not applicable**
(State or Other Jurisdiction of Incorporation or Organization)	(Primary Standard Industrial Classification Code Number)	(I.R.S. Employer Identification Number)

Jörn Aldag, Chief Executive Officer
Meibergdreef 61
Amsterdam 1105 BA, the Netherlands; Tel: +31 20 566 7394
(Address, Including ZIP Code, and Telephone Number,
Including Area Code, of Registrant's Principal Executive Offices)

Philip Astley-Sparke, President U.S. Operations
uniQure, Inc.
113 Hartwell Avenue
Lexington, MA 02421
Tel: +31 20 566 7394
(Name, Address, Including ZIP Code, and Telephone Number,
Including Area Code, of Agent for Service)

Copies to:

David E. Redlick, Esq.	**Eric W. Blanchard, Esq.**
Timothy J. Corbett, Esq.	**Brian K. Rosenzweig, Esq.**
WilmerHale LLP	**Covington & Burling LLP**
10 Noble Street	**The New York Times Building**
London EC2V 7QJ	**620 Eighth Avenue**
United Kingdom	**New York, New York 10018**
+44 20 7645 2400	**+1 212 841 1000**

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. o

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. o

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. o

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. o

CALCULATION OF REGISTRATION FEE

TITLE OF EACH CLASS OF SECURITIES TO BE REGISTERED	PROPOSED MAXIMUM AGGREGATE OFFERING PRICE[1][2]	AMOUNT OF REGISTRATION FEE[3]
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.
(3) Previously paid.

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.**

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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 17, 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. On or prior to completion of this offering, we intend to convert into a public company with limited liability (*naamloze vennootschap*), and our legal name will be uniQure N.V.

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.

	PER ORDINARY SHARE	TOTAL
Public Offering Price	$	$
Underwriting Discounts and Commissions		
Proceeds to uniQure before Expenses		

(1) 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 Partners**

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.

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, the "Management's Discussion and Analysis of Financial Condition and Results o Operations" section 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. W 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 fo 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 preclinica 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 the case of diseases for which relatively modest levels of gene expression may result in therapeutic benefit, we expect that we will be able to achieve adequate levels of expression using existing, naturally derived AAV serotypes. In the case of diseases for which higher levels of gene expression may be required for therapeutic benefit, however, we believe we may need access to more potent vectors thar are currently available. To complement our internal development efforts in this regard, in January 2014 we entered into a collaboration and license agreement with 4D Molecular Therapeutics, or 4D, a recently formed, private biotechnology company with a team that we believe is a leader in AAV vector discovery and optimization. 4D uses directed evolution techniques, which involve an iterative selection process in which researchers screen libraries of mutant AAV variants to identify those that are expected to have optimal properties for achieving higher levels of gene expression.

  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 tissue in non-human primates for more than five 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 ou 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.

LPLD is a serious, debilitating disease caused by mutations in the lipoprotein lipase, or LPL, gene, resulting in significant diminished or absent activity of the LPL protein and, as a consequence, severe hypertriglyceridemia. Severe hypertriglyceridemia results in hyper-chylomicronemia, or dramatic and potentially life-threatening increases in the level of 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.

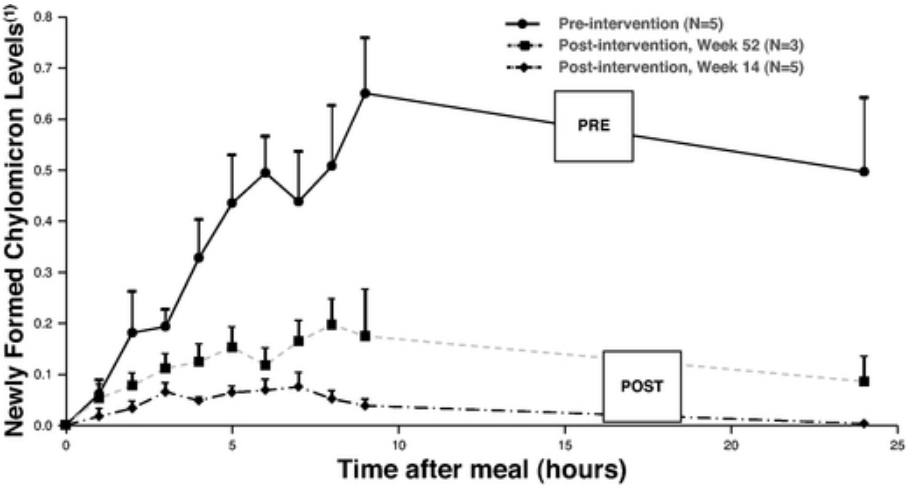
Glybera is designed to restore the LPL enzyme activity required by tissues of the body to clear, or process, the fat-carrying chylomicron particles that are formed in the intestine and transported via the blood to the muscle after a fat-containing meal. The product consists of an engineered copy of the 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 the three clinical studies, we did not observe a statistically significant reduction in triglyceride levels beyond 12 weeks, which was the primary efficac endpoint; however, 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, which was a secondary endpoint. The graph below depicts the appearance and removal of newl 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 remova 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 benef 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 second half 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.

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. The FDA advised that severe hypertriglyceridemia is currently considered a hallmark of LPLD, and agreed that changes in chylomicron metabolism following a meal is a relevant marker of biological activity of Glybera. However, the FDA also advised that changes in chylomicron metabolism following a meal alone would not be adequate for obtaining marketing approval in the United States at this stage, since it is not yet sufficiently understood how this biological effect translates into clinical meaningfulness. 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 trial.

We plan to discuss the details of the EU post-approval trial and patient registry with the FDA, and if applicable to seek to amend the protocols for the this post-approval trial and patient registry so that they could also serve as a clinical program with a design that addresses the FDA's requirements. We also 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 and registry. We believe the patient registry 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 further clinical trial of Glybera and the patient registry to file a BLA for Glybera with the FDA in 2017. 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.

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 followin chart provides summary information on the most advanced of these programs:

Product / Product Candidate	Vector	Gene	Indication	Collaborator	Development Stage	Comments
Internal Programs						
Glybera (E.U.)	AAV1	Lipoprotein Lipase (LPL)	LPLD	Chiesi	EU Commercial launch planned first half of 2014	• Post-approval study initiation in second half 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)[1]	Hemophilia B	Chiesi	Pre-Clinical / Phase I / II	• Phase I/II trial by St. Jude using AAV8 & uniQure's hFIX transgene ongoing • uniQure Phase I/II planned to commence second half of 2014
Collaborator Sponsored Programs						
AMT-021	AAV5	Porphobilinogen Deaminase[1]	Acute Intermittent Porphyria (AIP)	Digna Biotech (Licensor: CIMA)	Pre-Clinical	• 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[1]	AAV2	GDNF[1,2]	Parkinson's Disease	UCSF (Funder & Sponsor: NIH)	Pre-Clinical	• 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, wit 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 th gene therapy being used in this clinical trial. We understand that, to date, Digna has not observed a reduction in the urinary levels of toxi metabolites in trial participants that might have served as a surrogate marker for efficacy. We understand from Digna Biotech that clinica outcomes 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 a-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 have an agreement in principle with Institut Pasteur to acquire the clinical results and commercial rights under this program following completion of this Phase I/II clinical trial, and are currently in negotiations with Institut Pasteur regarding the terms of a definitive agreement in this regard. 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 earlier 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 developmer program. Based on the results of the UCSF program, we may decide to develop an AAV2-based gene therapy containing the GDNF gen 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 ou 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 in the 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 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.

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 shareholder 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. 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 will 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 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 in this prospectus;
- 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 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."

We may take advantage of these exemptions for up to five years following completion of this offering 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	We currently estimate that we will use the net proceeds from this offering, together with our cash on hand, as follows: • to complete the building out and equipping of our manufacturing facility in Lexington, Massachusetts; • 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; • to fund our planned Phase I/II clinical trial of AMT-060 in hemophilia B; • 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. See "Use of Proceeds" for additional information.
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:

- 8,459,274 ordinary shares issued under our equity incentive plans issuable upon the exercise of options outstanding as of the date of thi prospectus at a weighted average exercise price of €0.78 per share;
- up to ordinary shares reserved for future issuance under our equity incentive plans immediately following this offering;
- 3,048,728 ordinary shares issuable upon exercise of options granted on January 17, 2014 in connection with our collaboration and license agreement with 4D Molecular Therapeutics, at an exercise price of €0.01 per share; and

- 854,036 ordinary shares issuable upon the exercise of warrants outstanding as of the date of this prospectus 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 prio 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 audited 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:

	YEAR ENDED DECEMBER 31,		NINE MONTHS ENDED SEPTEMBER 30,	
€ in thousands (except per share data)	**2011**	**2012**	**2012**	**2013**
Revenues:				
License revenues	€ —	€ —	€ —	€ 220
Collaboration revenues	—	—	—	1,831
Total revenues	**—**	**—**	**—**	**2,051**
Cost of goods sold	—	—	—	(800)
Gross profit	**—**	**—**	**—**	**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)
Operating result	**(17,141)**	**(14,191)**	**(9,895)**	**(15,800)**
Finance income	277	22	**16**	**48**
Finance expense	(436)	(547)	(545)	(4,676)
Net loss	**€ (17,300)**	**€ (14,716)**	€ (10,424)	€ (20,428)
Basic and diluted loss per share	**€ (0.73)**	**€ (0.34)**	**€ (0.25)**	**€ (0.39)**
Weighted average shares outstanding used in computing per share amounts (in thousands):				
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 offerin 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:

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

(1) 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 als 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 shareholders' 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 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 our EMA-mandated post-approval clinical trial of Glybera and implement an LPLD patient registry;
- conduct a 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.

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 our ability 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, which 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 employee headcount as of December 31, 2013 was 87. 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. In addition, we have an agreement in principle with Institut Pasteur to acquire the clinical results and commercial rights under our Sanfilippo B program following completion of the ongoing Phase I/II clinical trial, and are currently in negotiations with Institut Pasteur regarding the terms of a definitive agreement in this regard. If we are unable to reach agreement with Institut Pasteur regarding such rights, we would have to conduct additional clinical development ourselves and may experience delays in progressing this clinical program. 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.

Because of the nature of the gene therapies we are developing, regulators may require us to demonstrate long-term gene expression or clinical efficacy, which may require longer clinical trial periods or longer patient follow-up than is typically required in the case of other therapies.

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 additional trials for Glybera or 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 results of our prior clinical trials of Glybera will not be sufficient to obtain FDA approval, and the FDA may not ultimately approve Glybera for marketing in the United States. 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 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 will seek to amend the protocol for our European Union post-approval trial of Glybera so that such trial also could serve as such a trial. The FDA may require preclinical testing or clinical trials beyond this 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 Committee for Advanced Therapeutics, or 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.

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, we are in discussions with the FDA regarding the regulatory pathway for Glybera in the United States, and must design an adequate and appropriately controlled clinical trial of Glybera to support a BLA filing. This could involve additional time, which may delay our U.S. clinical program for Glybera. 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 the 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 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 our other product candidates may be smaller than we or third parties currently project, which may affect the addressable markets for Glybera and our other 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 the facility we are currently building out in Lexington, Massachusetts. 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 personal 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 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 current Good Manufacturing Practices, or 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 managment, 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:

- 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.

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. 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. 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 this prospectus, 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 more 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.

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 prevented 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 experience 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 ordinary 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 NASDAQ Global Market, 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 the ordinary shares you purchase.

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 biotechnology 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 judgments 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 and directors 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 and management board under Dutch law are different than under the laws of some U.S. jurisdictions. In the performance of their duties, our supervisory board and management board are required by Dutch law to consider the interests of uniQure, its shareholders, its employees and other stakeholders and not only those of our shareholders.

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 of and receipt of data from 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 will we 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 option 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 together with our consolidated financial statements and the related notes and the "Management's Discussion and Analysis of Financial Condition and Results of Operations" section of this prospectus.

	AS OF SEPTEMBER 30, 2013	
(in thousands, except share and per share data)	**ACTUAL**	**AS ADJUSTED[1]**
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	€

(1) 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:

- 8,459,274 ordinary shares issued under our equity incentive plans issuable upon the exercise of options outstanding as of the date of this prospectus at a weighted average exercise price of €0.78 per share;
- up to ordinary shares reserved for future issuance under our equity incentive plans following this offering;
- 3,048,728 ordinary shares issuable upon exercise of options granted on January 17, 2014 in connection with our collaboration and license agreement with 4D Molecular Therapeutics, at an exercise price of €0.01 per share; and
- 854,036 ordinary shares issuable upon the exercise of warrants outstanding as of the date of this prospectus 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 ordinary share. Net tangible book value per ordinary share represents the amount of our total tangible assets less our total liabilities, divided by the number of ordinary 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 ordinary share ($ per ordinary share). This amount represents an immediate increase in net tangible book value of € per ordinary share ($ per ordinary share) to our existing shareholders and an immediate dilution in net tangible book value of € per ordinary share ($ per ordinary share), or % per ordinary 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:

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

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 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 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 ordinary share, as adjusted to give effect to this offering, would be $ per ordinary share, and the dilution in pro forma net tangible book value per ordinary 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 ordinary 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 ordinary 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:

- 8,459,274 ordinary shares issued under our equity incentive plans issuable upon the exercise of options outstanding as of the date of this prospectus at a weighted average exercise price of €0.78 per ordinary share;
- up to ordinary shares reserved for future issuance under our equity incentive plans following this offering;
- 3,048,728 ordinary shares issuable upon exercise of options granted on January 17, 2014 in connection with our collaboration and license agreement with 4D Molecular Therapeutics, at an exercise price of €0.01 per share; and
- 854,036 ordinary shares issuable upon the exercise of warrants outstanding as of the date of this prospectus at an exercise price of €2.02 per ordinary share.

If the underwriters exercise their option to purchase additional ordinary 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 ordinary shares held by new investors will be increased to ordinary 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 audited 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
Revenues:				
License revenues	€ —	€ —	€ —	€ 220
Collaboration revenues	—	—	—	1,831
Total revenues	—	—	—	2,051
Cost of goods sold	—	—	—	(800)
Gross profit	—	—	—	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)
Operating result	**(17,141)**	**(14,191)**	**(9,985)**	**(15,800)**
Finance income	277	22	16	48
Finance expense	(436)	(547)	(545)	(4,676)
Net loss	**€ (17,300)**	**€ (14,716)**	**€ (10,424)**	**€ (20,428)**
Basic and diluted loss per share	**€ (0.73)**	**€ (0.34)**	**€ (0.25)**	**€ (0.39)**
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:

(€ in thousands)	AS OF DECEMBER 31, 2010	2011	2012	AS OF 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

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 January 14, 2014, the exchange rate was €0.732 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
Year Ended December 31:				
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	0.725	0.753	0.724	0.783
2014 (through January 14)	0.725	0.753	0.724	0.783
Month Ended:				
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	0.725	0.731	0.724	0.739
January 2014 (through January 14)	0.732	0.734	0.732	0.736

This prospectus also contains amounts that we have paid or may be required to pay in Canadian dollars. On January 10, 2014, the exchange rate quoted by the Federal Reserve Bank of New York was C$1.092 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 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 €138.0 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 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;
- fund expenses in connection with our new collaboration with 4D Molecular Therapeutics;
- 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 December 31, 2013, we had a total of 87 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—Chiesi". 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 co-development agreement. See "Business—Strategic Collaboration—Chiesi Farmaceutici."

Strategic Collaboration: 4D Molecular Therapeutics

In January 2014, we entered into a collaboration and license agreement with 4D for the discovery and optimization of next-generation AAV vectors. Under this agreement, we have an exclusive license to 4D's existing and certain future know-how and other intellectual property for the delivery of AAV vectors to CNS or liver cells for the diagnosis, treatment, palliation or prevention of all diseases or medical conditions. Under this collaboration, the 4D team, including Dr. David Schaffer, 4D's co-founder and Professor of Chemical and Biomolecular Engineering at the University of California, Berkeley, will establish a laboratory, which we will fund, at a cost of approximately $3.0 million in aggregate over the next three years, to identify next generation AAV vectors. We are also required to make payments for pre-clinical, clinical and regulatory milestones under the collaboration as well as to pay single-digit royalties. In addition, we have granted options to purchase an aggregate of 3,048,728 class B ordinary shares in connection with this collaboration, and will recognize resulting share-based payment expense over the next three years. To the extent that the collaboration is successful, we may also incur additional third party costs in developing any product candidates and also in preparing, filing and prosecuting additional patent applications. See "Business—Strategic Collaboration—4D Molecular Therapeutics".

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 expected 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.

Cost of Goods Sold

Cost of goods sold includes 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 cost 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 cost of goods sold although no product sales occurred. No further payments will be made until sales of Glybera commence. 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 or 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 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 were 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
Total	**15,500**	**10,231**	**(34)**	**5,690**	**9,856**	**73**

* 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 another 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 our 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 additional development work to seek 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."

In addition, in connection with the collaboration and license agreement we entered into with 4D Molecular Therapeutics during January 2014, we will incur additional expenses as we fund a joint research effort with 4D. Further, we granted options to purchase an aggregate of 3,048,728 of our class B ordinary shares to two consultants who will be providing services to us in connection with that agreement. The fair value of these options will vest over a future service period, and will have a significant impact on our expenses recognized. Finally, to the extent certain clinical and regulatory milestones are met, we will make milestone payments to 4D. See "Business—Strategic Collaboration—4D Molecular Therapeutics."

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 exchange 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

	NINE MONTHS ENDED SEPTEMBER 30,		
(€ in thousands)	**2012**	**2013**	**CHANGE**
Revenues:			
License revenues	€ —	€ 220	—%
Collaboration revenues	—	1,831	—
Total revenues	**—**	**2,051**	**—**
Cost of goods sold	—	(800)	—
Gross profit	—	**1,251**	—
Other income	315	686	118
Expenses:			
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
Operating result	**(9,895)**	**(15,800)**	**60**
Finance income	16	48	200
Finance expense	(545)	(4,676)	758
Net loss	(10,424)	(20,428)	96

Revenues

License revenues of €0.2 million in the nine months ended September 30, 2013 related to the amortization of the upfront 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 Sanfilippo B collaboration. 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 of 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 in the European Union 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 our convertible loans and the venture loan, 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 %
Revenues:	€ —	€ —	—
License revenues	—	—	—
Collaboration revenues	—	—	—
Total revenues	—	—	—
Cost of goods sold	—	—	—
Gross loss	—	—	—
Other Income	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
Net loss	€ (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 our 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, 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 our convertible loans, which were 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, 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 €138.0 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:

	YEAR ENDED DECEMBER 31,		NINE MONTHS ENDED SEPTEMBER 30,	
(€ in thousands)	**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% increase 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. The decrease reflected the reduction in net loss before corporate income tax for 2012 compared to 2011, which in turn was due to 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 activity. In 2012 our net loss before corporate income tax was €14.7 million, a decrease of €2.6 million compared to 2011. In addition, changes in overall composition of our working capital balance also resulted in an overall reduction in the cash 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 million 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[1]	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
Total	**24,160**	**13,497**	**7,492**	**45,149**

(1) 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 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 and security 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;
- expenses in connection with our collaboration with 4D Molecular Therapeutics;
- 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 the Hercules Agreement, 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 the Hercules Agreement 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.

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

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 partially paid in the fourth quarter of 2013, with the remainder to be paid 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 flows in future periods.

	PAYMENTS DUE BY PERIOD				
(€ in thousands)	**LESS THAN 1 YEAR**	**BETWEEN 1 AND 2 YEARS**	**BETWEEN 2 AND 5 YEARS**	**MORE THAN 5 YEARS**	**TOTAL**
License maintenance obligations[1][2]	279	279	735	324	1,617
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
Total	2,437	3,676	6,191	324	12,628

(1) Annual license maintenance payments will be no longer payable following the expiration of the license payment obligations. Thereafter, we have a fully paid-up license.

(2) Amounts are paid annually in advance; to the extent that we could terminate the agreement prior to the date of the next maintenance payment, these maintenance fees are not recognized within research commitments in the notes to the financial statements.

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.

- Our obligations under the collaboration and license agreement with 4D Molecular Therapeutics, entered into in January 2014, to fund research and development activities at a cost of approximately $3.0 million in aggregate over the next three years and approximately $200,000 of licenses fees during the first year.

- Payments in relation to the lease of the Lexington facility. These payments begin seven 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 seven 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 (€7.5 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 31, 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, less an initial repayment made in the third quarter of 2013. 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 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 required to fulfill 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 ordinary 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 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 we have not otherwise been 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 September 30, 2013, the total amount of tax losses carried forward was €121.5 million.

We have a history of tax losses, and therefore 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 the 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 we 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 our gene therapies. 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 was required to 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 the date of this prospectus, 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[1]	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
October 1, 2013	33,765	0.614	2.68	N/A	2.47
January 17, 2014	3,048,728	0.01		N/A	

(1) 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 options which have been granted and remained outstanding as of the date of this prospectus, 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 the date of this prospectus 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 was €2.2 million.

The intrinsic value of all outstanding vested and unvested options as of January 31, 2014 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 the 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 December 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 reference 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 an 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 an 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.

Share Options Grant on October 1, 2013

On October 1, 2013, we granted options to purchase an aggregate of 33,756 ordinary shares at an exercise price of €0.614 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 €2.68 as of the grant date. We performed a DCF as of October 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 an estimated value per ordinary share of €2.68, this resulted in the fair value per option of €2.25 to €2.28. The key assumptions we used to arrive at the estimated fair value per option of each tranche of the grant included an estimated expected volatility of 70%, an expected life of 5.5 to 6.3 years, and a risk-free rate of 0.9% to 1.0% 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 from €0.614 to €2.68 was primarily due to passage of time, such that positive operating cash flows come nearer.

The grant was originally discussed with the participant in June 2012, but the option agreement was only concluded on October 1, 2013. As a result, the option is granted with an exercise price of €0.614 and other terms as originally proposed in June 2012. To reflect the start of the service commencement period as of April 5, 2012, related option expenses for the period April 5, 2012 to October 1, 2013 were recorded based on an estimated grant date fair value of the options. An adjustment to these estimated expenses was recorded as of October 1, 2013 to reflect the fair value of the granted options as determined at the formal grant date, October 1, 2013.

Share Options Grant on January 17, 2014

On January 17, 2014, we granted options to purchase an aggregate of 3,048,728 ordinary shares at an exercise price of €0.01 per share. These options were granted in connection with the collaboration and license agreement we entered into on that date with 4D Molecular Therapeutics, and were granted to two consultants who will be providing services to us in connection with that agreement. The options vest in three tranches, on October 1, 2014, January 31, 2015, and January 31, 2016. Our management board and supervisory board determined that the fair value of the ordinary shares as of the grant date was $, the midpoint of the price range set forth on the front cover of this prospectus.

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 have an agreement in principle with Institut Pasteur to acquire the clinical results and commercial rights under this program following completion of this Phase I/II clinical trial, and are currently in negotiations with Institut Pasteur regarding the terms of a definitive agreement in this regard. 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 target tissue in non-human primates for more than five 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.

In the case of diseases for which relatively modest levels of gene expression may result in therapeutic benefit, we expect that we will be able to achieve adequate levels of expression using existing, naturally derived AAV serotypes. In the case of diseases for which higher levels of gene expression may be required for therapeutic benefit, however, we may need access to more potent vectors than are currently available. To complement our internal development efforts in this regard, in January 2014 we entered into a collaboration and license agreement with 4D, a recently formed, private biotechnology company with a team that we believe is a leader in AAV vector discovery and optimization. 4D uses directed evolution techniques, which involve an iterative selection process in which researchers screen libraries of mutant AAV variants to identify those with optimal properties for achieving higher levels of gene expression.

Under our collaboration and license agreement with 4D, we have an exclusive license to 4D's existing and certain future know-how and other intellectual property for the delivery of AAV vectors to CNS or liver cells for the diagnosis, treatment, palliation or prevention of all diseases or medical conditions. Under this collaboration, the 4D team, including Dr. David Schaffer, 4D's co-founder and Professor of Chemical and Biomolecular Engineering at the University of California, Berkeley, has agreed to establish a laboratory, which we have agreed to fund, to identify product candidates for clinical development.

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 B 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:

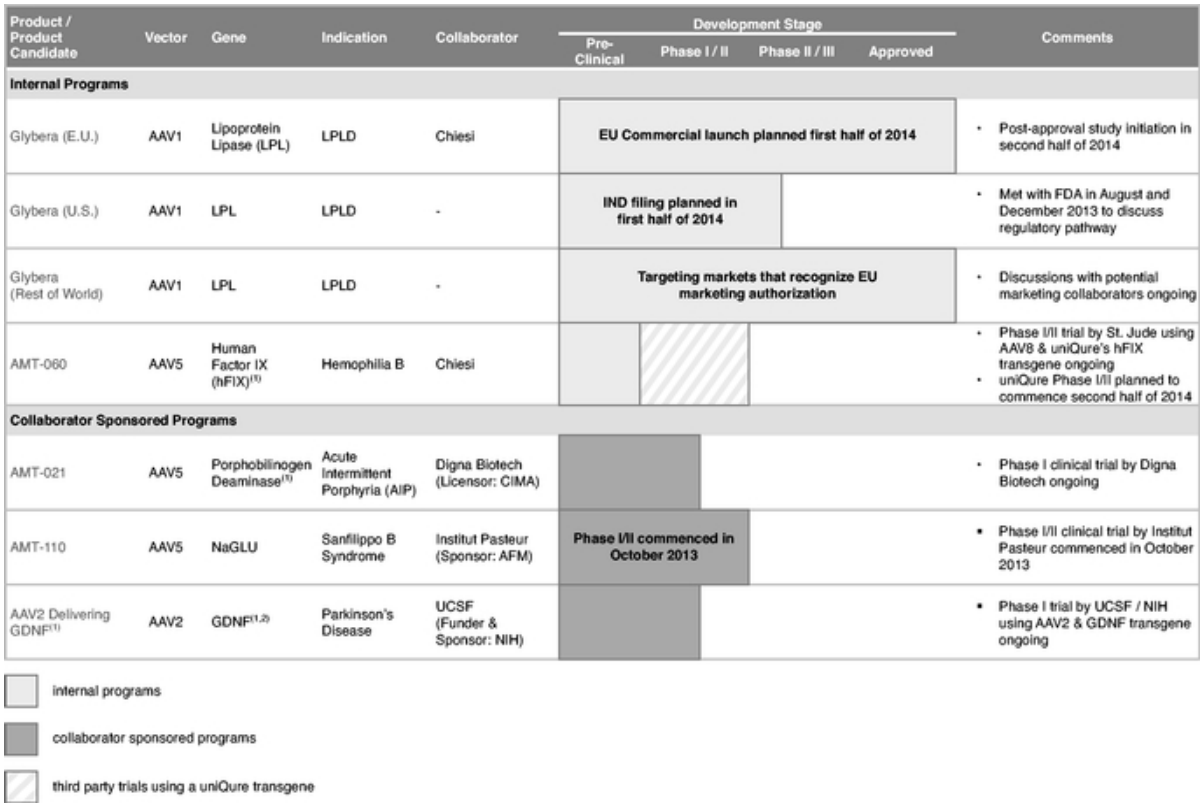
  - next-generation AAV vectors with the potential for increased gene expression through improved cell-specific delivery and efficient release of DNA in the cell nucleus;

  - 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:



(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 significant diminished or absent activity of the LPL protein and, as a consequence, severe hypertriglyceridemia. Severe hypertriglyceridemia results in hyper-chylomicronemia, or dramatic and potentially life-threatening increases in the level of 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.

LPLD is a rare disease. The medical literature generally states that the prevalence of LPLD is approximately one person per million people. However, we believe that this 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. 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 might be up to 4.6 persons with LPLD per million people (180 × 2.55%). Because of the small number of LPLD patients in the diagnostic study described above and the absence of other evidence our estimate is preliminary, and we plan to conduct additional studies to establish a more precise figure. 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 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.

The link among the missing LPL enzyme activity, measurable metabolic lipid dysregulation and the actual disease manifestations of LPLD is not fully understood. There is an association between higher than normal plasma triglyceride levels, impaired clearance of chylomicrons following a meal and pancreatitis. Our earlier clinical trials of Glybera aimed to demonstrate a relevant and sustainable reduction in plasma triglycerides. As the clinical development program progressed over the following years, however, we began studying the level of newly formed chylomicrons after a meal as a relevant biological marker of LPL activity.

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 $1x10^{12}$ genome copies per kilogram of body weight, which is a 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. We are currently discussing with the EMA an amendment to the protocol pursuant to which only half of the LPLD patients will also receive an immunosuppressant regimen for a period beginning three days and ending 12 weeks after Glybera administration. We expect that this will allow us to evaluate the adequacy of the immunosuppressant regimen. We anticipate that the trial will be conducted as a multicenter trial including sites in the United States, which we expect will enable us to enroll all patients during the first six to twelve months and to complete the study towards the end of 2016, including a two-year follow-up period. We will collect data on a broad range of clinically meaningful endpoints in this open label, single arm post-approval study, in which the current primary objective is to investigate the effect of Glybera on chylomicron metabolism after a meal in LPLD patients over a 2 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 second half of 2014. We will 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 half 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. The FDA advised that severe hypertriglyceridemia is currently considered a hallmark of LPLD, and agreed that changes in chylomicron metabolism following a meal is a relevant marker of biological activity of Glybera. However, the FDA also advised that changes in chylomicron metabolism following a meal alone would not be adequate for obtaining marketing approval in the United States at this stage, since it is not yet sufficiently understood how this biological effect translates into clinical meaningfulness. 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 trial.

We plan discuss the details of the EU post-approval trial and patient registry with the FDA, and if applicable to seek to amend the protocols for the this post-approval trial and patient registry so that they also could serve as a clinical program with a design that addresses the FDA's requirements. We also 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 and registry. We believe the patient registry 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 further clinical trial of Glybera and the patient registry to file a BLA for Glybera with the FDA in 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 non-controlled, 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 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
Retrospective Analysis:			
Case Note Review AMT-011-03	17	• Effect on frequency and severity of pancreatitis in patients treated with Glybera in prior clinical trials	Retrospective case note review of patients through 2010
Clinical Trials:			
Phase II/III trial AMT-011-02	5	Primary: • Effect on fasting triglyceride levels at 12 weeks Secondary: • Effect on chylomicron metabolism at 14 and 52 weeks • LPL activity at 3 months • Safety	1 year
Phase II/III trial AMT-011-01	14	• Safety • Effect on triglyceride levels at 12 weeks • LPL activity in the muscle at 6 months	5 years
Phase I/II trial AMT-010-01	8	• Safety • Effect on fasting triglyceride levels at 12 weeks	5 years

Efficacy. We did not achieve statistically significant data for the primary efficacy endpoints in the clinical trials described above.

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 Revised 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 primary objective of the study was to achieve a 40% reduction of mean fasting triglyceride levels at 12 weeks. In addition, a secondary objective of the 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. 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;
- 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.

Prior to the clinical trial, subjects participated in an observational study to establish baseline data.

Each patient received one intramuscular dose of 1×10^{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:

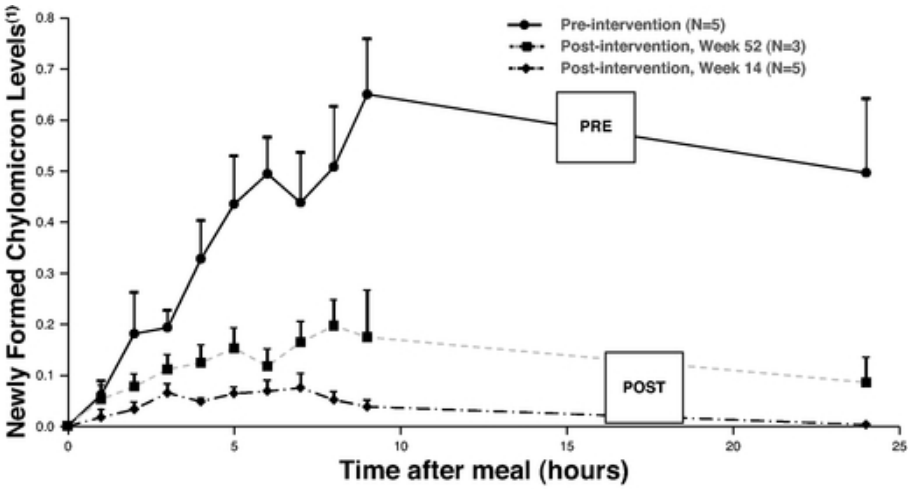
- only one of five patients demonstrated a fasting triglyceride level of less than 10 mmol/L;

- 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.

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.

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 activity 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 3×10^{11} gc/kg, and the third cohort of eight patients received a lower dose of 1×10^{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.

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 might have served 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 clinical outcomes 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 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 a-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 lysomal 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.

We have an agreement in principle with Institut Pasteur to acquire the clinical results and commercial rights under this program following completion of this Phase I/II clinical trial, and are currently in negotiations with Institut Pasteur regarding the terms of a definitive agreement in this regard.

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 Asklêpios 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 of $15,000 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 of $15,000 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 of $50,000 for each product up to an annual maximum of $150,000 and limited by an overall specified life-time maximum of $500,000 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 of $50,000 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 percentage 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 percentage 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 of $50,000 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 of $30,000, 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 of $100,000, 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 of $10,000 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 Applicada, 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: Chiesi

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, Kyrghyzstan, 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.

Strategic Collaboration: 4D Molecular Therapeutics

In January 2014, we entered into a collaboration and license agreement with 4D for the discovery and optimization of next-generation AAV vectors. Under this agreement, 4D has granted us an exclusive, worldwide license, with the right to grant sublicenses, to 4D's existing and certain future know-how and other intellectual property, including certain patent rights 4D has exclusively licensed from the Regents of the University of California, for the delivery of AAV vectors to CNS or liver cells for the diagnosis, treatment, palliation or prevention of all diseases or medical conditions. Under this collaboration, the 4D team, including Dr. David Schaffer, 4D's co-founder and Professor of Chemical and Biomolecular Engineering at the University of California, Berkeley, has agreed to establish a laboratory to identify next generation AAV vectors. In addition, in connection with our entry into this collaboration, Dr. Schaffer will join our Supervisory Board.

We have agreed to fund a three-year research collaboration, which can be extended at our option for an additional year, to be conducted under a mutually agreed research plan. We are entitled to select a specified number of AAV variants from the research collaboration. We will have exclusive rights to further research, develop, manufacture and commercialize the selected AAV variants, as well as AAV vectors and products containing such AAV variants, or licensed products, and 4D retains no rights to the selected AAV variants for any use. During the research collaboration and throughout the term of the agreement, 4D has agreed to work exclusively with us to research, develop, manufacture and commercialize AAV variants, AAV vectors and products containing AAV vectors for delivery to CNS or liver cells for the diagnosis, treatment, palliation or prevention of all diseases or medical conditions.

Our research collaboration with 4D is guided by a joint research steering committee. Subject to limitations specified in the agreement, if the joint research steering committee is not able to make a decision by consensus and the parties are not able to resolve the issue through escalation to specified senior executive officers of the parties, then we have final decision-making authority with respect to all matters except for certain matters that will be decided by an independent scientific panel, certain matters that will be decided by binding arbitration, and certain matters that require mutual agreement.

We and 4D must each use commercially reasonable efforts to conduct the research collaboration in accordance with the research plan. We must use commercially reasonable efforts to develop, manufacture and commercialize licensed products under the agreement. After the research collaboration ends, 4D may notify us from time to time as to one or more products that 4D or a third party proposes to develop and commercialize using rights that are exclusively licensed to us. If we are not, or are not interested in, researching or developing any such proposed product or a product that is competitive with such proposed product, then we may be required to negotiate and enter into a sublicense to a third party, or an amendment to the agreement, that grants such third party or 4D, respectively, the right to research, develop, manufacture and commercialize such proposed product on commercially reasonable terms.

Under the agreement, we have agreed to make a one-time upfront payment of $100,000 and another one-time payment of $100,000 upon the joint research steering committee's approval of the research plan, including an associated budget. Our payment obligations under the agreement include the research

collaboration funding described above as well as payments for the achievement of specified pre-clinical, clinical and regulatory milestones of up to $5,000,000 for each licensed product that we develop under the collaboration, which milestones are payable once for each indication for which the licensed product is developed. We have also agreed to pay 4D royalties equal to a single-digit percentage of net sales, if any, of licensed products by us or our affiliates. We will also pay 4D a double-digit percentage of any sublicensing income we receive, subject to a floor of a low single-digit percentage of net sales, if any, by sublicensees of certain licensed products. Our obligation to pay royalties expires on a product-by-product and country-by-country basis, upon the latest of:

- the expiration of the last valid claim of specified patent rights covering the relevant product in the relevant country;
- the expiration of any applicable exclusivity, including orphan drug status or data exclusivity, and any extension thereto, granted by a regulatory authority in the relevant country with respect to the relevant product; or
- 10 years after the first commercial sale of the relevant product in the relevant country.

Term and Termination. The agreement will remain in force until all of our payment obligations under the agreement expire or we or 4D exercise our rights to terminate it. Either party may terminate the agreement in the event of an uncured material breach by the other party, in the event the other party becomes subject to specified bankruptcy, insolvency or similar circumstances. 4D will be deemed in material breach under specified circumstances involving Dr. Shaffer's unavailability. If we materially breach the agreement in a manner that relates to a specific AAV vector or product, and not to the agreement as a whole, 4D may only terminate the agreement as to the indication for which such AAV vector or product was being developed. We may terminate the agreement for convenience after the research collaboration ends, subject to a specified notice period. We may also terminate the agreement at any point prior to the first anniversary of the effective date if the joint research steering committee determines that it would be futile to continue the research collaboration or that 4D is not making bona fide efforts to achieve the timelines in the research plan. Our research collaboration funding obligation continues for specified periods following certain terminations.

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 the 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:

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- completion of preclinical laboratory tests, animal studies and formulation studies in compliance with the FDA's current Good Laboratory Practice, or cGLP, regulations;
- submission to the 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 the 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 the 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 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 cGCP requirements. A protocol for each clinical trial and any subsequent protocol amendments must be submitted to the 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 the FDA unless before that time the 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 the 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 the 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 the 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 the 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 the FDA and certain state agencies. Both domestic and foreign manufacturing establishments must register and provide additional information to the 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 the 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 the 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 the FDA, the 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, the 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 the FDA to reconsider the application. If and when those deficiencies have been addressed to the FDA's satisfaction in a resubmission of the BLA, the FDA will issue an approval letter.

If the 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 the 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 the 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 the 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 the 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, the 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 the 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 the 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 the 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 the FDA generally will require approval or clearance of the diagnostic at the same time that the 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 CHMP 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, the 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 liabilty.

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 obilgations 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 MAHto 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 cGCP 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

Except as described below, 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 request for arbitration was received by the International Court of Arbitration at the International Chamber of Commerce on December 12, 2013, which represents the start date of the arbitration. 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 December 31, 2013, we had a total of 87 employees, of whom 29 had an M.D. or Ph.D. degree, or the foreign equivalent. Of these employees, 20 were engaged in research and development, seven 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[1]	TERM EXPIRES[2]
Ferdinand Verdonck	71	Member of the Supervisory Board (Chairman)	2012	2017
Sander Slootweg	45	Member of the Supervisory Board	2012	2015
Sander van Deventer	59	Member of the Supervisory Board	2012	2016
Joseph M. Feczko	64	Member of the Supervisory Board	2012	2016
François Meyer	65	Member of the Supervisory Board	2012	2015
David Schaffer[3]	44	Member of the Supervisory Board	*	*
Paula Soteropoulos	46	Member of the Supervisory Board	2013	2017

(1) For periods prior to 2012, certain of our directors served as directors of AMT, our predecessor entity.

(2) The expiration of terms listed in the table is expected be formally approved by the extraordinary general meeting of shareholders to be held on January 27, 2014, in accordance with the rotation plan adopted by the supervisory board. Terms expire as of the date of the annual general meeting of shareholders in the year listed.

(3) We intend to appoint David Schaffer to our supervisory board prior to the completion of this offering, for an initial term ending at the annual meeting of shareholders in 2016.

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 has been Professor of Translational Gastroenterology at the Leiden University Medical Center since 2008 and is a partner of Forbion Capital Partners, which he joined in 2006. He serves on the boards of Cardoz AS, Argos Biotherapeutics, gICare 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.

David Schaffer will join our supervisory board prior to the completion of this offering. Dr. Schaffer is Professor of Chemical and Biomolecular Engineering, Bioengineering, and Neuroscience at University of California Berkeley, a position he has held since 2007, as well as Director of the Berkeley Stem Cell Center since 2011. Dr. Schaffer is also co-founder of 4D Molecular Therapeutics, a company specializing proprietary technology for gene therapy products. We entered into a collaboration and license agreement with 4D Molecular Therapeutics in January 2014. Previously, Dr. Schaffer was Assistant Professor from 1999 to 2005 and Associate Professor from 2005 to 2007 at the University of California, Berkeley Department of Chemical Engineering & Helen Wills Neuroscience Institute. He serves on the boards of the American Society for Gene and Cell Therapy and the Society for Biological Engineering. He has more than 20 years of experience in chemical and molecular engineering, and stem cell and gene therapy research, has over 130 scientific publications, and serves on 5 journal editorial boards and 5 industrial scientific advisory boards. Dr. Schaffer holds a bachelor of science degree in chemical engineering from Stanford University and a Ph.D. in Chemical Engineering from the Massachusetts Institute of Technology. We believe Dr. Schaffer is qualified to serve on our supervisory board due to his extensive relevant scientific expertise and experience 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 has 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.

NAME	AGE	POSITION	DATE OF APPOINTMENT
Jörn Aldag	54	Chief Executive Officer	October 4, 2009
Piers Morgan	47	Chief Financial Officer	December 1, 2009

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 Ltd, 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 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 United States where he grew operations from scratch, including overseeing the construction of a commercial-grade manufacturing facility. Prior to the Biovex Group, 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, including 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 a 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. Prior to 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 United Kingdom.

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 three years, unless the resolution appointing a supervisory board member provides otherwise. The articles of association provide that the supervisory board members must retire periodically in accordance with a

rotation plan 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 be reappointed 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;
- investments requiring an amount equal to at least one fourth of our issued capital plus our reserves, as reflected in our most recent balance sheet;
- 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 Mr. Feczko (Chairman), Ms. Soteropoulos and Mr. Verdonck. Each member satisfies the independence requirements of the NASDAQ listing standards, and Mr. Verdonck 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. van Deventer (Chairman), Meyer and Verdonck. 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. van Deventer (Chairman), Meyer and Verdonck. 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 2013, the aggregate compensation paid to our supervisory directors was €441,000, consisting of the payments set forth below:

(€ in thousands)	SHARE-BASED PAYMENTS[1]	BOARD FEE	2013 TOTAL
Ferdinand Verdonck	244	38	282
Sander van Deventer[2]	—	—	—
Joseph Feczko	30	28	58
Francois Meyer	30	28	58
Sander Slootweg[3]	—	—	—
Paula Soteropoulos[4]	32	11	43
Total	**366**	**105**	**441**

(1) The share-based payment reflects the value of share options granted during the year, as required by IFRS.
(2) Dr. van Deventer receives no remuneration.
(3) Mr. Slootweg receives no remuneration.
(4) Appointed July 22, 2013.

Management Board and Other Senior Management Compensation

The table below sets out a breakdown of the compensation in 2013 of the members of the management board and senior management:

(€ in thousands)	SHORT TERM EMPLOYEE BENEFITS	SHARE-BASED PAYMENTS	POST-EMPLOYMENT BENEFITS	OTHER LONG TERM BENEFITS	TERMINATION BENEFITS	TOTAL
Jörn Aldag	480	266	41	—	—	787
Piers Morgan	267	111	19	—	—	397
Total for Management Directors	**747**	**377**	**60**	**—**	**—**	**1,184**
Senior Management	1,102	873	109	—	—	2,084
Total	**1,849**	**1,250**	**169**	**—**	**—**	**3,268**

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.

2014 Share Incentive Plan

Our 2014 Plan was adopted by our supervisory board and approved by our shareholders in January, 2014. We will begin making grants under the 2014 Plan following the effective date of the Registration Statement of which this prospectus forms a part. The 2014 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, directors, consultants and advisors are eligible to receive awards under the 2014 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 2014 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 2014 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 the management board to grant awards under our 2014 Plan, the management board will have the power to make awards to all of our employees, except the members of the management board 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 the management board, including the exercise price of such awards, and the maximum number of shares subject to awards that the management board 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 2014 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 2014 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 2014 Plan on or after , 2024. Our supervisory board may amend, suspend or terminate the 2014 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 2014 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
Coller 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

(1) 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[1]	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 managing directors, 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.[1]	588,235
Cooperatieve Gilde Healthcare II U.A.	882,353
Jörn Aldag	29,412
Piers Morgan	11,765

(1) 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 class A 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 class B 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 class C 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.

4D Molecular Therapeutics Collaboration

On January 17, 2014, we entered into a collaboration and license agreement with 4D Molecular Therapeutics, a company co-founded by Dr. David Schaffer, a nominee to our supervisory board who will be appointed prior to the completion of this offering. In connection with that transaction, we have agreed to provide specified research and development financing, are obligated to make certain upfront, royalty and milestone payments, and have granted an option to purchase up to 1,524,364 class B shares at an exercise price of €0.01 per share to Dr. Schaffer. See "Business—Strategic Collaboration—4D Molecular Therapeutics."

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 reclassification 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
5% Shareholders:			
Entities affiliated with Forbion[1]	21,673,522	35.6%	
Cooperatieve Gilde Healthcare II U.A.[2]	8,255,020	13.5%	
Entities affiliated with Advent[3]	3,761,612	6.2%	
Coller International Partners V-A, L.P.[4]	27,119,066	44.5%	
Chiesi Farmaceutici S.p.A.[5]	5,546,070	9.1%	
Management Board Members, Supervisory Board Members and Senior Management			
Ferdinand Verdonck[6]	609,542	1%	
Sander Slootweg[7]	21,673,522	35.6%	
Sander van Deventer[8]	21,673,522	35.6%	
Joseph M. Feczko[9]	242,116	*	
François Meyer[10]	192,133	*	
Paula Soteropoulos	—	—	
Jörn Aldag[11]	1,048,758	1.7%	
Piers Morgan[12]	526,303	*	
Philip Astley-Sparke[13]	21,673,522	35.6%	
Christian Meyer	—	—	
Harald Petry[14]	390,411	*	
Hans Preusting[15]	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 Coöperative 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 Coller International Partners V-A, L.P., or Coller; (ii) warrants held by Coller 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. Coller is a limited partner of the Forbion funds. Coller 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 Coller is Coller International General Partner V, L.P. of which Coller Investment Management Limited, or CIML, is the general partner. The directors of CIML are Jeremy Joseph Coller, 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 Coller. The CIML directors disclaim beneficial ownership of such ordinary shares except to the extent of their pecuniary interest therein. The address of Coller is c/o Coller 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 €3,000,000, divided into ordinary shares, each with a nominal value of €0.06, 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 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.

We will list our ordinary shares in registered form and such shares will not be certificated. We have appointed Computershare Trust Company, N.A. as our agent in New York to maintain our shareholders register and to act as transfer agent, registrar and paying agent for the ordinary shares. Our ordinary shares will be traded on the NASDAQ Global Market in book-entry form.

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 venootschap*) 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 or its nominee will be recorded in the shareholders register as the holder of the ordinary shares.

Corporate objectives

Under our 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 three years. A supervisory director may be reappointed for a term of up to three 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.

The agenda for a general meeting of shareholders must 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 10% 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 30 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 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 18 months 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 Netherlandsche 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 does not purport 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 U.S. 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, or 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 certain U.S. holders that are individuals, 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, or 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 2013 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 Partners 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:

UNDERWRITER	NUMBER OF ORDINARY SHARES
Jefferies LLC	
Leerink Partners LLC	
Piper Jaffray & Co.	
Total	

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 reallow, 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 reallowance 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-l(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 Partners LLC.

This restriction terminates after the close of trading of the ordinary shares on and including the 180th day after the date of this prospectus.

Jefferies LLC and Leerink Partners 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	$	9,660
FINRA filing fee	$	12,500
Nasdaq listing fee	$	125,000
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 currently 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, 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.uniqure.com. Information contained in or connected to our website is not a part of this prospectus.

UNIQUE B.V.

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UNIQURE B.V.

Unaudited Condensed Consolidated Balance Sheets
(€ in thousands)

	NOTE	DECEMBER 31, 2012	SEPTEMBER 30, 2013
Assets			
Non-current assets			
Intangible assets	8	3,278	6,770
Property, plant and equipment	7	1,185	1,353
Other non-current assets	9	—	917
Total non-current assets		4,463	9,040
Current assets			
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
Total current assets		1,104	34,631
Total assets		5,567	43,671
Equity			
Share capital		483	609
Share premium		114,795	142,444
Other reserves		1,508	5,924
Accumulated deficit		(117,234)	(137,656)
Total equity	13	(448)	11,321
Liabilities			
Non-current liabilities			
Borrowings	15	—	7,291
Financial lease liabilities	15	450	342
Deferred revenue	16	—	15,899
Total non-current liabilities		450	23,532
Current liabilities			
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
Total Current Liabilities		**5,565**	**8,818**
Total liabilities		6,015	32,350
Total equity and liabilities		5,567	43,671

The notes are an integral part of these condensed consolidated financial statements.

UNIQURE 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
Total revenues		**—**	**2,051**
Cost of goods sold	23	—	(800)
Gross profit		**—**	**1,251**
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)
Total Operating Costs		**(9,895)**	**(17,051)**
Operating result		**(9,895)**	**(15,800)**
Finance income		16	48
Finance expense	15	(545)	(4,676)
Finance income/(expense)—net		**(529)**	**(4,628)**
Result before corporate income taxes		**(10,424)**	**(20,428)**
Corporate income taxes		—	—
Net Loss		(10,424)	(20,428)
Other comprehensive income	19	—	6
Total comprehensive loss*		**(10,424)**	**(20,422)**
Loss per share attributable to the equity holders of the Company during the year			
Basic and diluted loss per share	21	(0.25)	(0.39)

* Total comprehensive loss is fully attributable to equity holders of the group

The notes are an integral part of these condensed consolidated financial statements.

UNIQURE B.V.

Unaudited Condensed Consolidated Statement of Changes in Equity
(€ in thousands)

	TOTAL SHARE CAPITAL	SHARE PREMIUM	OTHER RESERVES	ACCUMULATED DEFICIT	TOTAL EQUITY
Balance at January 1, 2012	237	99,947	2,728	(105,505)	**(2,593)**
Result for the period				(10,424)	**(10,424)**
Capital contributions	241	14,579			**14,820**
Share based payment/expense			1,228		**1,228**
Adjustment to reserves on expiration of the AMT option scheme			(2,987)	2,987	**—**
Balance at September 30, 2012	478	114,526	969	(112,942)	**3,031**
Result for the period				(4,292)	**(4,292)**
Capital contributions	5	269			**274**
Share-based payment/expense			539		**539**
Balance at December 31, 2012	483	114,795	1,508	(117,234)	**(448)**
Result for the period				(20,428)	**(20,428)**
Other Comprehensive Income				6	**6**
Capital contributions	126	27,649			**27,775**
Result on conversion of loan			3,005		**3,005**
Share-based payment/expense			1,411		**1,411**
Balance at September 30, 2013	609	142,444	5,924	(137,656)	**11,321**

The notes are an integral part of these condensed consolidated financial statements.

UNIQURE B.V.

Unaudited Condensed Consolidated Statement of Cash Flows
(€ in thousands)

	NOTE	NINE MONTHS ENDED SEPTEMBER 30, 2012	2013
Cash flow from operating activities			
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)
Net cash used in operating activities		(8,579)	1,674
Cash flow from investing activities			
Purchases of property, plant and equipment	7,12	(149)	(536)
Purchases of intangible assets	8	(386)	(3,647)
Interest received		108	4
Net cash used in investing activities		(427)	(4,179)
Cash flow from financing activities			
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		—
Net cash generated from financing activities		9,433	33,663
Net increase in cash, cash equivalents, and other bank overdrafts		427	31,158
Currency effect cash and cash equivalents		—	6
Cash, cash equivalents, and other bank overdrafts at beginning of the period		1,100	263
Cash, cash equivalents, and other bank overdrafts cash at end of the period	12	**1,527**	**31,427**

The notes are an integral part of these condensed consolidated financial statements.

UNIQURE 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:

Company name	*Formerly known as*
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[1]	Stichting participatie AMT[1]
uniQure Inc.[2]	

(1) 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.

(2) In May 2013 the Company incorporated uniQure Inc., a Delaware corporation and wholly owned subsidiary of uniQure biopharma B.V.

UNIQURE 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)

The unaudited condensed consolidated financial statements were authorized for issue by the supervisory board on December 19, 2013.

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:

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[(1)]	Levies

(1) 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.

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.

UNIQURE 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.

UNIQURE 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)			
At December 31, 2012				
Borrowings (excl. finance lease liabilities)	—	—	—	—
Financial lease liabilities	151	450	—	—
Debt to related party	1,618	—	—	—
Trade and other payables	3,916	—	—	—
Total	5,685	450	—	—
At September 30, 2013				
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	—	—	—
Total	7,739	2,855	4,778	

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)				
Assets as per balance sheet					
Receivables from related parties	26	—	—	—	26
Trade and other receivables	815	—	—	—	815
Cash and cash equivalents	263	—	—	—	263
Total	**1,104**	**—**	**—**	**—**	**1,104**

UNIQURE B.V.

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)			
Liabilities as per balance sheet				
Debt to related party	132	—	1,366	1,498
Financial lease liabilities	—	—	601	601
Trade and other payables	—	—	3,916	3,916
Total	**132**	**—**	**5,883**	**6,015**

	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)			
Assets as per balance sheet					
Receivables from related parties	726	—	—	—	726
Trade and other receivables	2,051	—	—	—	2,051
Cash and cash equivalents	31,427	—	—	—	31,427
Total	**34,204**	**—**	**—**	**—**	**34,204**

	LIABILITIES AT FAIR VALUE THROUGH PROFIT AND LOSS	DERIVATIVES USED FOR HEDGING	OTHER FINANCIAL LIABILITIES AT AMORTIZED COST	TOTAL
	(€ in thousands)			
Liabilities as per balance sheet				
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
Total	**1,165**	**—**	**14,360**	**15,525**

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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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
At December 31, 2012				
Debt to related party—embedded derivative (warrants)	—	—	132	**132**
Borrowings—embedded derivative (warrants)	—	—	—	**—**
	—	—	132	**132**

	LEVEL 1	LEVEL 2	LEVEL 3	TOTAL
At September 30, 2013				
Debt to related party—embedded derivative (warrants)	—	—	892	**892**
Borrowings—embedded derivative (warrants)	—	—	273	**273**
	—	—	1,165	**1,165**

	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	**1,165**
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.

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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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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)		
Period ended September 30, 2013					
Opening net book amount	598	—	270	317	1,185
Additions	—	85	55	426	566
Depreciation charge	(148)	—	(94)	(156)	(398)
Closing net book amount	450	85	231	587	1,353
At September 30, 2013					
Cost	1,264	85	3,014	1,305	5,668
Accumulated depreciation	(814)	—	(2,783)	(718)	(4,315)
Net book amount	450	85	231	587	1,353

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.

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8. Intangible Assets

	INTANGIBLE ASSETS (€ in thousands)
Period ended September 30, 2013	
Opening net book amount	3,278
Additions	3,647
Reductions	(155)
Amortization charge	—
Closing net book amount	6,770
At September 30, 2013	
Cost	6,770
Accumulated amortization and impairment	—
Net book amount	6,770

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
Trade and other receivables	**841**	**2,777**

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

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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
Inventories	**—**	**427**

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)	
Cash at bank and on hand	263	31,427

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 Coller 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.

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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:

	A	B	C	TOTAL
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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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:

Date	Description	Sub-class of ordinary shares	Number of shares	Share capital Amounts	Share premium Amounts	Total equity Amounts
				(€ 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
September 30,2012			**47,821,716**	**478**	**114,526**	**115,004**
November–December, 2012	Employees and other persons new equity investment	B	445,777	5	269	274
December 31, 2012			**48,267,493**	**483**	**114,795**	**115,278**
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
September 30, 2013			**60,948,978**	**609**	**142,444**	**143,053**

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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)		
2012					
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
September 30, 2012			**9,500**	**5,320**	**14,820**
November-December, 2012	Employees and other persons new equity investment	B	274	—	274
December 31, 2012			**9,774**	**5,320**	**15,094**
2013					
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
September 30, 2013			**14,278**	**13,497**	**27,775**

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.

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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

	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
Total trade and other payables	4,067	6,574

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.

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15. Borrowings

	DECEMBER 31, 2012	SEPTEMBER 30, 2013
	(€ in thousands)	
Non-current		
Borrowings	—	7,291
Finance lease liabilities	450	342
Total non-current	450	7,633
Current		
Debt to related party—Financial liability	1,366	—
Debt to related party—Embedded derivative	132	892
Borrowings—Embedded derivative	—	273
Total current	1,498	1,165
Total	1,948	8,798

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 Coller 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,

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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 though 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

	DECEMBER 31, 2012	SEPTEMBER 30, 2013
	(€ in thousands)	
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

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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.

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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 12 months ended December 31, 2013, employee payroll headcount increased from 50 to 87, 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.

	NINE MONTHS ENDED SEPTEMBER 30,	
	2012	2013
	(€ in thousands)	
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

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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.

	DECEMBER 31, 2012	SEPTEMBER 30, 2013
	(€ in thousands)	
Share options		
Total	8,031,777	8,451,110

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 Coller 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 Coller 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.

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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	ADVISORS FEES	TERMINATION BENEFITS	TOTAL
				(€ in thousands)			
Year ended December 31, 2012	Supervisory Board	—	134	—	121	—	**255**
	Managing directors	695	509	92	—	—	**1,296**
	Senior Management	689	452	41	—	—	**1,182**
		1,384	**1,095**	**133**	**121**	**—**	2,733
9 months ended September 30, 2012	Supervisory Board	—	79	—	93	—	**172**
	Managing directors	428	337	69	—	—	**834**
	Senior Management	488	297	31	—	—	**816**
		916	**713**	**100**	**93**	**—**	1,822
9 months ended September 30, 2013	Supervisory Board	—	211[2]	—	49	—	**260**
	Managing directors	577[1]	325	45	—	—	**947**
	Senior Management	753	335	78	—	—	**1,166**
		1,330	**871**	**123**	**49**	**—**	2,373

(1) The Management board received Management bonuses
(2) 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).

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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
Total	2,169	1,762

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	—	—
Total	277	298

UNIQURE 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

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

UNIQURE B.V.

Consolidated Balance Sheets
(€ in thousands)

	NOTES	AS AT DECEMBER 31, 2010	2011	2012
Assets				
Non-current assets				
Intangible assets	*(6)*	2,916	2,725	3,278
Property, plant and equipment	*(7)*	1,286	895	1,185
Total Non-current assets		**4,202**	**3,620**	**4,463**
Current assets				
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
Total Current assets		**18,501**	**2,184**	**1,104**
Total assets		**22,703**	**5,804**	**5,567**
Equity				
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)
Total equity	*(10)*	**13,659**	**(2,593)**	**(448)**
Liabilities				
Non-current liabilities				
Financial lease liabilities	*(11)*	221	180	450
Debt to related party	*(12, 24)*	4,621	4,544	—
Non-current liabilities		**4,842**	**4,724**	**450**
Current liabilities				
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
Total Current liabilities		4,202	3,673	5,565
Total liabilities		**9,044**	**8,397**	**6,015**
Total equity and liabilities		**22,703**	**5,804**	**5,567**

The accompanying notes form an integral part of the consolidated financial statements.

UNIQURE B.V.

Consolidated Statements of Comprehensive Income
(€ in thousands, except share data and per share data)

	NOTE	YEAR ENDED DECEMBER 31, 2011	2012
Revenues:		€—	€—
License revenues		—	—
Collaboration revenues		—	—
Total revenues		**—**	**—**
Cost of goods sold		—	—
Gross loss		**—**	**—**
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)
Total operating costs	*(15)*	**(19,333)**	**(14,840)**
Operating result		**(17,141)**	**(14,191)**
Finance income	*(17)*	277	22
Finance expense	*(17)*	(436)	(547)
		(159)	**(525)**
Result before corporate income taxes		(17,300)	(14,716)
Corporate income taxes	*(18)*	—	—
Net loss (Attributable to equity holders of the Company)		**(17,300)**	**(14,716)**
		(17,300)	**(14,716)**
Other comprehensive income		—	—
Total comprehensive loss*		**(17,300)**	**(14,716)**
Loss per share attributable to the equity holders of the Company during the year			
Basic and diluted loss per share	***(19)***	**(0.73)**	**(0.34)**

* Total comprehensive loss is fully attributable to equity holders of the Company.

The accompanying notes form an integral part of the consolidated financial statements.

UNIQURE 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
Balance at January 1, 2011	*(5)*	**235**	**99,841**	**1,788**	**(88,205)**	**13,659**
Result for the year		—	—	—	(17,300)	(17,300)
Capital contributions	*(5)*	2	106	—	—	108
Share-based payment expenses		—	—	940	—	940
Balance at December 31, 2011	*(5)*	**237**	**99,947**	**2,728**	**(105,505)**	**(2,593)**
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
Balance at December 31, 2012		**483**	**114,795**	**1,508**	**(117,234)**	**(448)**

The accompanying notes form an integral part of the consolidated financial statements.

UNIQURE B.V.

Consolidated Statements of Cash Flow
(€ in thousands)

	NOTES	YEAR ENDED DECEMBER 31, 2011	2012
Cash flow from operating activities			
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)
Net cash used in operating activities		**(16,705)**	(11,277**)**
Cash flow from investing activities			
Purchases of property, plant and equipment	*(7)*	(200)	(392)
Purchases of intangible assets	*(6)*	(109)	(553)
Interest received	*(17)*	147	113
Net cash used in investing activities		**(162)**	(832**)**
Cash flow from financing activities			
Capital contribution from shareholders	*(10, 21)*	108	9,774
Convertible loans drawn down	*(12)*	—	1,498
Net cash generated from financing activities		**108**	11,272
Net decrease in cash, cash equivalents and other bank overdrafts		**(16,759)**	(837**)**
Cash, cash equivalents and bank overdrafts at the beginning of the year	*(9)*	**17,859**	1,100
Cash, cash equivalents at the end of the year	*(9)*	**1,100**	263

The accompanying notes form an integral part of the consolidated financial statements.

UNIQURE 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 lipoproteinlipase 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.

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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).

UNIQURE B.V.

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.

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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, Coller International Partners V-A, L.P. ("Coller 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
- Coller Capital
- Forbion

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- 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 supervisory board 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

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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:

COMPANY NAME	FORMERLY KNOWN AS
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.

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(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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- 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.

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(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.

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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.

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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.

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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.

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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.

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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 been 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.

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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.

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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.

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(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.

	AS OF DECEMBER 31,			
	2011		2012	
(€ in thousands)	AMOUNT	CREDIT RATING (MOODY'S)	AMOUNT	CREDIT RATING (MOODY'S)
Bank				
Rabo Bank	1,088	AAA	258	AA2
Van Lanschot	5	A-*	5	A-*
Deutsche Bank	7	A2	—	n/a
Total	**1,100**		**263**	

* 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.

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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, Coller Capital.

	LESS THAN 1 YEAR	BETWEEN 1 AND 2 YEARS	BETWEEN 2 AND 5 YEARS	OVER 5 YEARS
	(€ in thousands)			
December 31, 2012				
Trade and other payables	4,067	450	—	—
Debt to related party	1,618	—	—	—
Total	**5,685**	**450**	**—**	**—**
December 31, 2011				
Trade and other payables	3,673	180		—
Debt to related party	250	250	5,250	—
Total	**3,923**	**430**	**5,250**	**—**

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	TOTAL
	(€ in thousands)				
Assets as per balance sheet					
Trade and other receivables	841	—	—	—	841
Financial assets at fair value through profit and loss	—	—	—	—	—
Cash and cash equivalents	263			—	263
Total	**1,104**	**—**	**—**	**—**	**1,104**

	LIABILITIES AT FAIR VALUE THROUGH PROFIT AND LOSS	DERIVATIVES USED FOR HEDGING	OTHER FINANCIAL LIABILITIES AT AMORTIZED COST	TOTAL
	(€ in thousands)			
Liabilities as per balance sheet				
Debt to related party	132	—	1,366	1,498
Finance lease liabilities	—	—	601	601
Trade and other payables		—	3,916	3,916
Total	**132**	**—**	**5,883**	**6,015**

	DECEMBER 31, 2011				
	LOANS AND RECEIVABLES	ASSETS AT FAIR VALUE THROUGH PROFIT AND LOSS	DERIVATIVES USED FOR HEDGING	AVAILABLE FOR SALE	TOTAL
	(€ in thousands)				
Assets as per balance sheet					
Trade and other receivables	1,084	—	—	—	1,084
Cash and cash equivalents	1,100			—	1,100
Total	**2,184**	**—**	**—**	**—**	**2,184**

	LIABILITIES AT FAIR VALUE THROUGH PROFIT AND LOSS	DERIVATIVES USED FOR HEDGING	OTHER FINANCIAL LIABILITIES AT AMORTIZED COST	TOTAL
	(€ in thousands)			
Liabilities as per balance sheet				
Debt to related party	2	—	4,542	4,544
Finance lease liabilities	—	—	221	221
Trade and other payables		—	3,632	3,632
Total	**2**	**—**	**8,395**	**8,397**

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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.

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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

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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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.

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Notes to Consolidated Financial Statements

Assets and liabilities

	AT DECEMBER 31, 2011		
	PER AMT CONSOLIDATED 2011 AUDITED ACCOUNTS	ADJUSTMENT	2011 RESTATED FOR UNIQURE CONSOLIDATED ACCOUNTS
	(€ in thousands)		
Assets			
Non-current assets			
Intangible assets	—	2,725	2,725
Property, plant and equipment	—	895	895
Non-current assets	**—**	**3,620**	**3,620**
Current assets			
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)	—
Current assets	**5,804**	**(3,620)**	**2,184**
Total assets	**5,804**	**—**	**5,804**
Liabilities			
Non-current liabilities			
Financial lease liabilities	—	180	180
Debt to related party	—	4,544	4,544
	—	**4,724**	**4,724**
Current liabilities			
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)	—
	8,397	**(4,724)**	**3,673**
Total liabilities	**8,397**	**—**	**8,397**

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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 UNIQURE CONSOLIDATED ACCOUNTS
	(€ in thousands, except share data and per share data)		
Other income	—	2,192	2,192
Total operating profit	**—**	**2,192**	**2,192**
Research and development costs	—	(15,500)	(15,500)
General and administrative costs	—	(3,781)	(3,781)
Other losses—net		(26)	(26)
Total operating costs	**—**	**(19,038)**	**(19,038)**
Operating result	—	(17,116)	(17,116)
Finance income	—	277	277
Finance costs	—	(462)	(462)
Result before corporate income taxes	—	(17,300)	(17,300)
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	—
Result for the year	**(17,300)**	**—**	**(17,300)**
Attributable to:			
Ordinary shareholders of the Company	**(17,300)**	**—**	**(17,300)**
Loss per share for result attributable to the equity holders of the Company during the year			
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	—
Basic and diluted loss per share	**(0.73)**	**—**	**(0.73)**

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Notes to Consolidated Financial Statements

Consolidated Statement of Changes in Equity

	PER AMT CONSOLIDATED 2011 AUDITED ACCOUNTS	ADJUSTMENT	2011 RESTATED FOR UNIQURE CONSOLIDATED ACCOUNTS
		(€ in thousands)	
Balance at December 31, 2010			
Share capital	940	(705)	235
Share premium	99,136	705	99,841
Total	**100,076**	**—**	**100,076**
Capital contributions			
Share capital	10	(8)	2
Share premium	98	8	106
Total	**108**	**—**	**108**
Balance at December 31, 2011			
Share capital	950	(713)	237
Share premium	99,234	713	99,947
Total	**100,184**	**—**	**100,184**

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Cash flow for the period

	PER AMT CONSOLIDATED 2011 AUDITED ACCOUNTS	ADJUSTMENT (€ in thousands)	2011 RESTATED FOR UNIQURE CONSOLIDATED ACCOUNTS
Cash flow from operating activities			
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)
Net cash used in continuing operating activities	**—**	**(16,705)**	**(16,705)**
Net cash used in discontinued operating activities	**(16,705)**	**16,705**	
Net cash used in operating activities	**(16,705)**	**—**	**(16,705)**
Cash flow from investing activities			
Purchases of property, plant and equipment		(200)	(200)
Purchases of intangible assets		(109)	(109)
Interest received		147	147
Net cash used in continuing investing activities		**(162)**	**(162)**
Net cash used in discontinued investing activities	**(162)**	**162**	**—**
Net cash used in investing activities	**(162)**	**—**	**(162)**
Capital contribution from shareholders			108
Net cash generated from continuing financing activities	**—**	**108**	**108**
Net cash generated from discontinued financing activities	108	**(108)**	**—**
Net cash generated from financing activities	108	**—**	**108**
Net decrease in cash, cash equivalents and other bank overdrafts of continuing activities	**—**	**(16,759)**	**(16,759)**
Net decrease in cash, cash equivalents and other bank overdrafts of discontinued activities	**(16,759)**	**16,759**	**—**
Net decrease in cash, cash equivalents and other bank overdrafts of continuing and discontinued activities	**(16,759)**	**—**	**(16,759)**
Cash, cash equivalents and bank overdrafts at the beginning of the year	17,859	—	17,859
Cash, cash equivalents at the end of the year (not classified as assets held for sale)	**—**	**1,100**	**1,100**
Cash, cash equivalents at the end of the year (classified as assets held for sale)	**1,100**	**(1,100)**	**—**
Cash, cash equivalents at the end of the year	**1,100**	**—**	**1,100**

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Notes to Consolidated Financial Statements

6. Intangible Assets

(€ in thousands)	LICENSES
At January 1, 2011	
Cost	3,216
Accumulated amortization and impairment	(300)
Net book amount	**2,916**
Year ended December 31, 2011	
Opening net book amount	2,916
Additions	109
Amortization and impairment charge	(300)
Closing net book amount	**2,725**
At December 31, 2011	
Cost	3,025
Accumulated amortization and impairment	(300)
Net book amount	**2,725**
Year ended December 31, 2012	
Opening net book amount	2,725
Additions	553
Amortization and impairment charge	—
Closing net book amount	3,278
At December 31, 2012	
Cost	3,278
Accumulated amortization and impairment	—
Net book amount	3,278

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
Total	**2,725**	**3,278**

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-licence 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-licence 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 Rafaelle 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 Rafaelle 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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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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7. Property, Plant and Equipment

	LEASEHOLD IMPROVEMENT	LABORATORY EQUIPMENT	COMPUTEER HARDWARE/ SOFTWARE	TOTAL
		(€ in thousands)		
As of January 1, 2011				
Cost	721	2,839	504	4,064
Accumulated amortization and impairment	(385)	(1,963)	(430)	(2,778)
Net book amount	**336**	**876**	**74**	**1,286**
Year ended December 31, 2011				
Opening net book amount	336	876	74	1,286
Additions	49	100	51	200
Depreciation charge	(123)	(414)	(54)	(591)
Closing net book amount	**262**	**562**	**71**	**895**
As of December 31, 2011				
Cost	770	2,939	555	4,264
Accumulated amortization and impairment	(508)	(2,377)	(484)	(3,369)
Net book amount	**262**	**562**	**71**	**895**
Year ended December 31, 2012				
Opening net book amount	262	562	71	895
Additions	494	20	324	838
Depreciation charge	(158)	(312)	(78)	(548)
Closing net book amount	**598**	**270**	**317**	**1,185**
As of December 31, 2012				
Cost	1,264	2,959	879	5,102
Accumulated amortization and impairment	(666)	(2,689)	(562)	(3,917)
Net book amount	**598**	**270**	**317**	**1,185**

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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8. Trade and Other Receivables

(€ in thousands)	2011	2012
Receivables from related parties (Note 24)	**35**	**26**
VAT to be received	249	418
Tax on wages to be received	—	—
Social Security to be received	—	—
Total taxes and social securities	**249**	**418**
Accounts receivable	2	0
Interest to be received	121	2
Prepaid expenses	—	—
Other receivables	677	395
Other receivables and prepayments	**800**	**397**

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	—
	1,100	**263**

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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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 UNIQURE CAPITAL *(BASED ON SHARES OF €0.01 NOMINAL VALUE)*
Share capital (ordinary shares)			
As of January 1, 2011	**23,512,225**		
Share capital		940	235
Share premium		99,136	99,841
Total		**100,076**	**100,076**
New shares issued	**235,902**		
Share capital		10	2
Share premium		98	106
Total		**108**	**108**
As of December 31, 2011	**23,748,127**		
Share capital		950	237
Share premium		99,234	99,947
Total		**100,184**	**100,184**
New shares issued prior to April 5, 2012	**7,352,938**		
Share capital		294	74
Share premium		2,206	2,426
Total		**2,500**	**2,500**
Shares in issue at April 5, 2012	**31,101,065**		
Share capital		1,244	311
Share premium		101,440	102,373
Total		**102,684**	**102,684**
New shares issued after April 5, 2012	**17,166,428**		
Share capital		n/a	172
Share premium		n/a	12,422
Total		**n/a**	**12,594**
As of December 31, 2012	**48,267,493**		
Share capital		n/a	483
Share premium		n/a	114,795
Total		**n/a**	**115,278**

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

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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:

	A	B	C	TOTAL
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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	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
		9,774	**5,320**	**15,094**

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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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
Number of options outstanding as of January 1	**1,354,150**	**1.95 - 2.92**	**1,898,200**	**1.95 - 2.92**
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
Number of options outstanding as of December 31	**1,898,200**	**1.95 - 2.92**	8,031,777	0.614

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 WEIGHTED AVERAGE REMAINING CONTRACTUAL LIFE	RANGE EXERCISE PRICE IN EUR PER SHARE	OPTIONS
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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YEAR ENDED DECEMBER 31, 2011 WEIGHTED AVERAGE REMAINING CONTRACTUAL LIFE	RANGE EXERCISE PRICE 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
At December 31, 2011	**1.95 - 2.92**	**1,898,200**

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.

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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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- 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)
Total	**221**	**601**

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	—	—
Total	**221**	**601**

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

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 Coller 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 though 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.

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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
	4,544	**1,498**

13. Trade and Other Payables

Trade and other payables are as follows:

	2011	2012
	(€ in thousands)	
Trade payables	**1,736**	**2,099**
Payables to related parties	**—**	**1,366**
Wage taxes	653	130
Accrued social security costs	60	21
Social security and other taxes	**713**	**152**
Short-term lease liabilities	41	151
Accrued expenses	833	1,204
Other amounts to be paid	350	461
Other current liabilities	**1,224**	**1,816**

The carrying values of trade and other payables are assumed to approximate their fair values.

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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:

	2011	2012
	(€ in thousands)	
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
	19,334	**14,840**

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.

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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)	
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
	8,492	**8,350**
Number of employees at the end of the period	85	67

17. Finance Income and Cost

	2011	2012
	(€ in thousands)	
Finance income:		
Interest income current accounts	70	22
Derivative result	207	—
	277	**22**
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)
	(435)	**(547)**
Finance costs—net	**(158)**	**(525)**

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)
Tax charge	—	—

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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
Basic loss per share	**(0.73)**	**(0.34)**

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	—	—
	108	**9,774**

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	—	—
	2,067	**2,169**

UNIQURE 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	—	—
	343	**277**

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

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 in 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.

UNIQURE B.V.

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:

	SALARY	BONUS	SHARE-BASED PAYMENTS[1]	PENSIONS	ADVISOR'S FEE	2012 TOTAL	2011 TOTAL
				(€ in thousands)			
Ferdinand Verdonck	—	—	14	—	29	43	37
Sander van Deventer[2]	—	—	—	—	8	8	56
Joseph Feczko	—	—	40	—	29	69	27
Edwin de Graaf[3]	—	—	—	—		—	—
Francois Meyer	—	—	40	—	29	69	27
Sander Slootweg[3]	—	—	—	—	—	—	—
Philippe Van Holle[4]	—	—	40	—	26	66	27
Steven Holtzman[5]	—	—	—	—	—	—	—
Total	—	—	134	—	121	255	174

(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:

DECEMBER 31, 2012	SHORT TERM EMPLOYEE BENEFITS	SHARE-BASED PAYMENTS[1]	POST-EMPLOYMENT BENEFITS	OTHER LONG TERM BENEFITS	TERMINATION BENEFITS	TOTAL
			(€ in thousands)			
Jörn Aldag	437	359	64	—	—	860
Piers Morgan	258	150	28	—	—	436
Total for Management Directors	**695**	**509**	**92**	**—**	**—**	**1,296**
Senior Management	689	452	41	—	—	1,182
Total	**1,384**	**961**	**133**	**—**	**—**	**2,478**

(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).

UNIQURE B.V.

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:

DECEMBER 31, 2011	SHORT TERM EMPLOYEE BENEFITS	SHARE-BASED PAYMENTS[1]	POST-EMPLOYMENT BENEFITS	OTHER LONG TERM BENEFITS	TERMINATION BENEFITS	TOTAL
			(€ in thousands)			
Jörn Aldag	390	267	57	—	—	714
Piers Morgan	227	186	17	—	—	430
Total for Management Directors	**617**	**453**	**74**	**—**	**—**	**1,144**
Senior Management	403	271	41	—	—	715
Total	**1,020**	**724**	**115**	**—**	**—**	**1,859**

(1) The share-based payment reflects the value of options granted during the year.

Shares and Share Options Held by Key Management

Options

	NUMBER OF OPTIONS AT JANUARY 1, 2012	OPTIONS GRANTED DURING THE YEAR	OPTIONS LAPSED/EXPIRED DURING THE YEAR	NUMBER OF OPTIONS AT DECEMBER 31, 2012
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
Total	**833,000**	**5,204,125**	**(833,000)**	**5,204,125**

Depositary receipts

	NUMBER OF DEPOSITARY RECEIPTS FOR SHARES[1]
Jörn Aldag	196,945
Piers Morgan	109,712
Senior Management	15,776
Total	**322,433**

(1) These Depositary 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
Total	**35**	**26**

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	2012
	(€ in thousands)	
Audit fees Annual Report	167	65
Audit fees Half-Year Report	46	—
Tax and HR advisory services	39	5
Total	**252**	**70**

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.

UNIQURE 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 B.V.

PRELIMINARY PROSPECTUS

Jefferies
Leerink Partners

Piper Jaffray & Co.

, 2014

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

Awards under our 2012 Stock Option Plan

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.

DATE OF GRANT	NUMBER OF SHARES UNDERLYING SHARE OPTIONS	CURRENT EXERCISE PRICE PER SHARE
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
October 1, 2013	33,756	€ 0.614

Other option awards

On January 17, 2014, we granted options to purchase an aggregate of 3,048,728 class B ordinary shares at an exercise price of €0.01 per share. These options were granted in connection with the collaboration and license agreement we entered into on that date with 4D Molecular Therapeutics, and were granted to two consultants who will be providing services to us in connection with that agreement. This issuance was made to U.S. persons pursuant to Section 4(2) of the Securities Act.

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 Amendment No. 1 to its registration statement to be signed on its behalf by the undersigned, thereunto duly authorized, on January 17, 2014.

UNIQURE B.V.

By: /s/ PIERS MORGAN

Name: **Piers Morgan**
Title: **Chief Financial Officer and Managing Director**

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.

SIGNATURES	TITLE	DATE
/s/ * **Jörn Aldag**	Chief Executive Officer *(Principal Executive Officer)*	January 17, 2014
/s/ PIERS MORGAN **Piers Morgan**	Chief Financial Officer *(Principal Financial and Accounting Officer)*	January 17, 2014
/s/ * **Ferdinand Verdonck**	Chairman	January 17, 2014
/s/ * **Sander Slootweg**	Non-Executive Director	January 17, 2014
/s/ * **Sander van Deventer**	Non-Executive Director	January 17, 2014
/s/ * **Joseph M. Feczko**	Non-Executive Director	January 17, 2014
/s/ * **François Meyer**	Non-Executive Director	January 17, 2014
/s/ * **Paula Soteropoulos**	Non-Executive Director	January 17, 2014

***By:** /s/ PIERS MORGAN

Name: **Piers Morgan**

SIGNATURES	TITLE	DATE
Title: **Attorney-in-Fact** **UNIQURE INC.** Authorized Representative in the United States		
By: /s/ PHILIP ASTLEY-SPARKE		
Name: **Philip Astley-Sparke** Title: **President, US Operations**		January 17, 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, Academish 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 Asklêpios 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 Applicada, 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+	2014 Share Incentive Plan
10.23+	Form of Incentive Share Option Agreement under the 2014 Share Incentive Plan
10.24+	Form of Share Option Agreement under the 2014 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.
10.32†	Collaboration and License Agreement, dated January 17, 2014, by and between uniQure biopharma B.V. and 4D Molecular Therapeutics, LLC.

Exhibit No.	Description
10.33	Option Agreement, dated January 17, 2014, by and between the Registrant and Dr. David Kirn
10.34	Option Agreement, dated January 17, 2014, by and between the Registrant and Dr. David Schaffer
10.35	Commitment Letter pursuant to Collaboration Agreement, dated January 17, 2014, by the Registrant and acknowledged and agreed by 4D Molecular Therapeutics, LLC, Dr. David Schaffer and Dr. David Kirn
21.1**	Subsidiaries of the Registrant
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
99.2	Consent of David Schaffer to be named as a director of the Registrant

* 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

** Previously filed

Exhibit 8.1

WILMERHALE

+1 212 295 6827 (t)
+1 212 230 8888 (f)
wilmerhale.com

, 2014

UniQure B.V.
Meibergdreef 61
Amsterdam 1105 BA
The Netherlands

Re: Ordinary Shares of UniQure B.V.

Ladies and Gentlemen:

In connection with the public offering of ordinary shares, par value €0.01 per share (the "ordinary shares") of UniQure B.V. (the "Company"), pursuant to the registration statement on Form F-1 under the Securities Act of 1933, as amended (the "Securities Act"), originally filed by the Company with the Securities and Exchange Commission (the "Commission") on January 2, 2014 (File No. 333-193158) (as so filed and as amended, the "Registration Statement"), you have requested our opinion concerning the statements in the Registration Statement under the heading "Taxation—Taxation in the United States."

In connection with rendering the opinion set forth below, we have examined and relied on the Registration Statement and such other documents as we have deemed necessary or relevant as a basis for the opinion set forth below. We have not independently verified any factual matters.

For purposes of rendering our opinion, we have assumed the authenticity of original documents, the accuracy of copies, the genuineness of all signatures and the legal capacity of all persons executing all instruments or documents examined or relied on by us.

Our opinion is based upon the relevant provisions of the United States Internal Revenue Code of 1986, as amended (the "Code"), the Treasury Regulations promulgated thereunder, and interpretations of the foregoing as expressed in court decisions and administrative determinations, all as in effect on the date of this opinion and all of which are subject to change at any time (possibly with retroactive effect). A change in the authorities upon which our opinion is based could affect the conclusions expressed herein. We undertake no obligation to update or supplement this opinion to reflect any changes of law or fact.

Our opinion is not binding upon the Internal Revenue Service (the "IRS") or any court. Thus, no assurance can be given that a position taken in reliance on our opinion will not be challenged by the IRS or rejected by a court.

On the basis of and subject to the foregoing and in reliance on the assumptions described above, subject to the limitations set forth in the Registration Statement, the statements of law or legal conclusions in the Registration Statement under the heading "Taxation—Taxation in the United States" constitute the opinion of Wilmer Cutler Pickering Hale and Dorr LLP as to the material United States federal income tax consequences to U.S. holders (as defined therein) of the acquisition, ownership and disposition of ordinary shares.

This opinion is limited to the matters of federal income tax law of the United States set forth in the Registration Statement, and we express no opinion with respect to any other federal, state, local or foreign tax issues, consequences or matters related to the acquisition, ownership and disposition of the ordinary shares.

This opinion is furnished to you solely in connection with the Registration Statement and may not be relied upon by anyone else or used for any other purpose without our prior written consent, provided, however, that it may be relied upon by persons entitled to rely on it pursuant to applicable provisions of federal securities laws.

We hereby consent to the filing of this opinion as Exhibit 8.1 to the Registration Statement and to the use of our name under the caption "Legal Matters" in the Registration Statement. In giving such consent, we do not thereby admit that we are within the category of persons whose consent is required under Section 7 of the Securities Act or the rules or regulations of the Commission promulgated thereunder, nor do we thereby admit that we are experts with respect to any part of such Registration Statement within the meaning of the term "experts" as used in the Securities Act or the rules and regulations of the Commission promulgated thereunder.

Very truly yours,

WILMER CUTLER PICKERING HALE AND DORR LLP

By: _____
Richard E. Andersen, Partner

Confidential Materials omitted and filed separately with the
Securities and Exchange Commission. Double asterisks denote omissions.

SUBLICENSE AND RESEARCH AGREEMENT

This Agreement is made and entered into this 18th day of June, 2001 by and between **Xenon Genetics Inc.**, a corporation organized a d existing under the laws of Canada, with registered offices at Suite 100 - 2386 East Mall, Vancouver, BC, Canada V6T 1Z3 (hereinafter referred to as: "**Xenon**"), of the one part, 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, (hereinafter referred to as "**AMT**"), of the other part, the parties (hereinafter also individually referred to as "**Party**" and collectively as "**Parties**");

WITNESSETH

WHEREAS AMT is conducting research and development programs in the area of gene therapy and has extensive research and development capabilities, including production facilities, to investigate and develop new therapeutics for use in the area of gene therapy;

WHEREAS, the University of British Columbia (hereinafter referred to as: "**UBC**") has expertise with respect to lipoprotein lipase (hereinafter referred to as "**LPL**") specifically in the area of diagnostics, animal models, clinical genetics and gene therapy, such research programs headed by Dr. Michael Hayden;

WHEREAS for many years, UBC and Academic Hospital at the University of Amsterdam ("**AMC**") have an ongoing research collaboration with respect to LPL, such programs headed by their respective principal researchers Dr. John Kastelein from AMC and Dr. Michael Hayden from UBC;

WHEREAS UBC and AMC wish to extend their scientific collaboration in the area of LPL gene therapy in humans including all research and development required for establishing a successful clinical LPL gene therapy protocol, with commercial aspects, for their mutual benefit;

WHEREAS AMT has expressed its interest in becoming responsible for the following: the total commercial operation, including development of the preclinical and clinical program, production of preclinical and clinical products, all current intellectual property rights of the

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Parties in the LPL gene therapy field, as well as improvements, variations, updates, modifications and enhancements to the existing intellectual property rights that will result from the joined activities of the Parties in this field;

WHEREAS AMC has granted an exclusive license to AMT with respect to its intellectual property rights in the area of LPL gene therapy in humans, including, *(pending receipt of the final approval of UBC, in accordance with the Letter of Intent between UBC and AMC in that regard*), all AMC's rights relating to UBC-Amsterdam Technology (as defined hereafter) of which invention Dr. Michael Hayden and Dr. Kastelein are the inventors, including PCT Application #CA00/00762, and all patents and patent applications accruing therefrom;

WHEREAS UBC owns the intellectual property, including patents related to the UBC Technology, as defined hereinafter, and also, jointly with [**], patents related to the UBC-[**] Technology, as defined hereinafter, and also, jointly with AMC, patents related to the UBC-Amsterdam Technology, as defined hereinafter;

WHEREAS on August 1, 2000 Xenon entered into a Collaborative Research Agreement with UBC, pursuant to which UBC shall perform research projects for Xenon by making use of the know how and information it has developed relating to the technologies described above;

WHEREAS on August 1, 2000, Xenon also entered into a License Agreement with UBC, pursuant to which UBC granted to Xenon an exclusive license to the UBC Technology and, *(pending receipt of the approvals from [**] and AMC, respectively*), pursuant to which UBC also granted to Xenon an exclusive license to its part of the UBC-[**] Technology, and to its part of the UBC-Amsterdam Technology, with the right to grant sublicenses;

WHEREAS AMT wishes to acquire and Xenon wishes to grant to AMT, a sublicense under the License Agreement on the terms and conditions set forth in this Agreement;

WHEREAS AMT and Xenon have already agreed that AMT shall sponsor the research Project as defined in the Collaborative Research Agreement between Xenon and UBC and, for that purpose, AMT and Xenon entered into Heads of Agreement effective August 1, 2000;

WHEREAS the Parties now desire to come to a definitive agreement and to replace the Heads of Agreement by this Sublicense and Research Agreement.

NOW, THEREFORE, in consideration of the mutual covenants and promises set forth herein, and other good and valuable consideration the receipt and sufficiency of which is hereby acknowledged the Parties hereby agree as follows:

ARTICLE 1 - DEFINITIONS

1.1 Plural used in this Agreement shall mean singular and vice versa.

1.2 When used in this Agreement, the following terms shall mean:

(a) "**Affiliate**" means any person, corporation, limited liability company, partnership or other legal entity, if any, which is controlled by a Party or which is under common control with a Party. Such entity shall be regarded as controlling another entity if it owns or controls at least fifty percent (50%) of the shares of the subject entity entitled to vote in the election of directors (or, in the event of an entity that is not a corporation, for the election of the corresponding managing authority);

(b) "**AMC**" means the Academic Hospital at the University of Amsterdam, at Amsterdam, the Netherlands;

(c) **[**]**;

(d) "**Collaborative Research Agreement**" means the Collaborative Research Agreement dated August 1, 2000 between Xenon and UBC, attached to this Agreement as Annex 1, including any amendments thereto;

(e) "**Contract Period**" means the time period defined in the Collaborative Research Agreement;

(f) "**Effective Date**" means August 1, 2000;

(g) "**Field of Use**" means gene therapy being [**];

(h) "**Joint Improvements**" has the meaning set out in the License Agreement;

(i) "**License Agreement**" means the form of License Agreement attached to this Agreement as Annex 2, which, except for the expunged portions (which are confidential between Xenon and UBC) is identical to the License Agreement dated August 1, 2000 executed between Xenon and UBC, including any amendments thereto;

(j) "**Licensed Product(s)**" means a Product using or made by a process using the Xenon Licensed Rights;

(k) "**[**] Model**" means the [**] Model which is in the possession of Xenon and/or UBC;

(l) "**Net Sales**" means with respect to any Xenon Licensed Technology or Licensed Product, [**]. Where any Net Sales are derived from a country other than Canada it shall be converted to the equivalent in Canadian dollars on the date AMT is deemed to have received such Net Sales pursuant to the terms hereof at the rate of exchange set by the Bank of Montreal for buying such currency. The amounts of Canadian dollars pursuant to such conversion shall be included in Net Sales.

(m) "**Party**" "**Parties**" means Xenon or AMT or both, as appropriate;

(n) "**Patents**" has the meaning set out in the License Agreement;

(o) "**Product(s)**" has the meaning set out in the License Agreement;

(p) "**Project**" means the project description entitled "LPL Gene Therapy for LPL Deficiency", which is attached to the Collaborative Research Agreement as Appendix A, including any amendments thereto which the Parties and UBC may mutually agree to, from time to time, in accordance with Section 2.1 of the Collaborative Research Agreement;

(q) "**Term**" means the time period defined in Section 17.1 of the License Agreement;

(r) "**UBC**" means the University of British Columbia at Vancouver, BC, Canada;

(s) "**UBC-[**] Technology**" has the meaning set out in the License Agreement;

(t) "**UBC-Amsterdam Technology**" has the meaning set out in the License Agreement;

(u) "**UBC Improvements**" has the meaning set out in the License Agreement;

(v) "**UBC Technology**" has the meaning set out in the License Agreement;

(w) "**Xenon Licensed Technology**" means the Technology and any and all Improvements (as defined in the License Agreement), to the extent such are licensed to Xenon under Section 3.1 or Section 3.2 of the License Agreement; and

(x) "**Xenon Licensed Rights**" means the right, license and privilege granted to Xenon under Section 3.1 and Section 3.2 of the License Agreement.

ARTICLE 2 - COLLABORATIVE RESEARCH

2.1 AMT agrees to assume in their entirety, and to be fully and solely responsible and liable for the performance of the following responsibilities, covenants and obligations of Sponsor under the Collaborative Research Agreement:

(a) all Sponsor responsibilities under Section 3.3 of the Collaborative Research Agreement;

(b) all Sponsor responsibilities under Section 3.4(d) and Section 3.4(e) of the Collaborative Research Agreement;

(c) all Sponsor responsibilities under Section 4.2 of the Collaborative Research Agreement;

(d) all Sponsor responsibilities under Section 4.3 of the Collaborative Research Agreement;

(e) all Sponsor responsibilities under Article 5.0 of the Collaborative Research Agreement;

(f) all Sponsor responsibilities under Article 6.0 of the Collaborative Research Agreement;

(g) all Sponsor responsibilities under Article 7.0 of the Collaborative Research Agreement;

(h) all Sponsor responsibilities under Section 8.4 of the Collaborative Research Agreement;

(i) all Sponsor responsibilities under Section 9.5 of the Collaborative Research Agreement;

(j) all Sponsor responsibilities under Section 10.3 of the Collaborative Research Agreement;

(k) all Sponsor responsibilities under Section 10.4 of the Collaborative Research Agreement;

(l) all Sponsor responsibilities under Article 11 of the Collaborative Research Agreement;

(m) all Sponsor responsibilities under Article 12 of the Collaborative Research Agreement;

(n) all Sponsor responsibilities under Article 13 of the Collaborative Research Agreement;

(o) all Sponsor responsibilities under Article 14 of the Collaborative Research Agreement; and

(p) all Sponsor responsibilities under Article 15 of the Collaborative Research Agreement.

2.2 All Parties confirm that Section 2.1 herein comprises a complete list of all Sponsor responsibilities, covenants and obligations under the Collaborative Research Agreement, with the sole exception of those responsibilities and obligations under Section 4.1 of the Collaborative Research Agreement.

2.3 AMT further agrees to forward payment in full, in advance, to Xenon in the amounts and in the time frames shown in the following schedule:

Payment Due Date		Amount Due (CDN$)
On execution of this Agreement	$	75,000.00
November 1, 2000	$	75,000.00
February 1, 2001	$	75,000.00
May 1, 2001	$	75,000.00
August 1, 2001	$	75,000.00
November l 2001	$	75,000.00
February 1, 2002	$	75,000.00
May 1, 2002	$	75,000.00
Total	**$**	**600,000.00**

2.4 Xenon reserves the right to suspend work on the Project, or to immediately terminate the Project and this Agreement upon delivering written notice of same to AMT if AMT fails to make the aforementioned payments within thirty (30) days of the dates herein specified in Section 2.3 above.

2.5 To facilitate AMT's assumption of the Collaborative Research Agreement responsibilities and obligations defined in Section 2.1 above, Xenon agrees as follows:

(a) in a timely manner, Xenon will fulfill all obligations towards UBC, arising from the Collaborative Research Agreement, unless Xenon is unable to fulfill such obligations in a timely manner, or at all, due to a breach by AMT of this Agreement;

(b) in the event UBC provides Xenon with a copy of a proposed publication or presentation pursuant to Section 7.1 of the Collaborative Research Agreement, Xenon will promptly forward such copy to AMT;

(c) in the event UBC provides Xenon with notification pursuant to Section 8.4 of the Collaborative Research Agreement regarding the conception of intellectual property in the performance of the Project, Xenon will promptly forward a copy of such notification to AMT; and

(d) any notices or reports provided by UBC to Xenon under the Collaborative Research Agreement will be promptly forwarded to AMT.

2.6 In consideration of AMT's assumption of responsibilities and obligations under Sections 2.1 and 2.3 herein, Xenon agrees to transfer to AMT any rights or benefit Xenon may accrue as Sponsor during the Contract Term under Sections 2.2, 8.2, 8.3, and 8.4, and Article 3 and Article 9 of the Collaborative Research Agreement.

2.7 Xenon shall not terminate the Collaborative Research Agreement for whatever reason, unless AMT has given its prior written consent thereto. Notwithstanding the foregoing, Xenon may terminate the Collaborative Research Agreement if AMT is in breach of this Agreement.

2.8 Any amendment, deletion or addition of any provisions of the Collaborative Research Agreement to be made during the Contract Period, requires the prior written consent of AMT.

ARTICLE 3 - SUBLICENSE

3.1 In consideration of the sub-license fees, milestone payments and royalty payments reserved herein, and the covenants of AMT contained herein, Xenon hereby grants to AMT within the Field of Use, and AMT accepts, the exclusive worldwide right, sublicense and privilege under the Xenon Licensed Rights to use the Xenon Licensed Technology and to use, manufacture, distribute and sell Licensed Products (the "**Sublicense**").

3.2 Xenon shall not terminate the License Agreement unless AMT has given its prior consent thereto, such consent not to be unreasonably withheld. Notwithstanding the foregoing, Xenon may terminate the License Agreement, without AMT's consent, if AMT is in breach of this Agreement.

3.3 Any alteration, deletion or addition of any provisions of the License Agreement to be made during the Term requires the prior written consent of AMT, such consent not to be unreasonably withheld.

3.4 This Sublicense is personal to AMT.

3.5 AMT may not grant a further sublicense to a third party for the purpose of developing, marketing, selling, manufacturing or distributing Xenon Licensed

Technology or Licensed Products, unless Xenon and UBC have given their prior written consent thereto, such consent not to be unreasonably withheld. Notwithstanding the foregoing, AMT shall not be obligated to obtain UBC's consent to the granting of a sub-sublicense if the proposed sub-sublicense has a market capitalization in excess of CAN$[**] at the time of the granting of the sub-sublicense.

3.6 AMT shall deliver to Xenon a copy of each sub-sublicense granted within 30 days after execution.

3.7 AMT may register this Agreement with the relevant patent authorities in those jurisdictions in which AMT carries on business. At AMT's request, Xenon and UBC will provide reasonable assistance to AMT with respect to such registrations, provided that all reasonable costs incurred by UBC or Xenon in association with such registrations, including all legal expenses, shall be paid for by AMT. UBC and Xenon will, on request by AMT, endeavour to provide an estimate of such costs.

ARTICLE 4 - ASSIGNMENT

4.1 The Parties shall not be entitled to assign, transfer, mortgage, charge or otherwise dispose of any or all of the rights, duties or obligations granted to it under this Agreement, to any third party, unless the other Party and UBC have given their prior written permission thereto, such consent not to be unreasonably withheld. Said permission however, will not be required in the event AMT assigns this Agreement to an Affiliate, or assigns all of its business or substantially all of its business to a third party, as part of a merger, acquisition or other business combination.

4.2 Notwithstanding AMT's agreement to perform certain responsibilities, covenants and obligations of Xenon under the Collaborative Research Agreement, nothing in this Agreement shall be deemed to be an assignment of the Collaborative Research Agreement by Xenon.

ARTICLE 5 - PAYMENTS

5.1 Upon the Date of Execution, AMT shall pay to Xenon a non-refundable initial sub-license fee of CAN$75,000.00 (seventy-five thousand Canadian Dollars).

5.2 AMT shall reimburse Xenon:

(a) for the patent and legal costs incurred by Xenon before the Effective Date, such costs not to exceed CAN$80,000.00 (eighty thousand Canadian Dollars); and

(b) for all patent and legal costs incurred by Xenon after the Effective Date related to the preparing, filing, prosecuting or maintaining of patent(s) or patent application(s) sublicensed to AMT under Section 3.1 above.

These amounts will be paid to Xenon within [**] days after AMT's receipt of the pertaining specified invoices from Xenon, accompanied by copies of the relevant invoices of patent attorneys and any other third parties concerned. As of the date of execution of this Agreement, Xenon agrees to consult with AMT prior to incurring any and all significant patent and legal costs, and obtain AMT's consent prior to authorizing its patent agents and/or patent counsel to incur said costs (whenever reasonably possible given the circumstances and timelines involved), such consent which shall not be unreasonably withheld.

5.3 In addition to the payments, referred to in Sections 2.3, 5.1 and 5.2, AMT shall make the following payments to Xenon on the achievement of the following specified milestones:

(a) [**] CAN$ ([**] Canadian Dollars) to be paid within [**] days after [**]; and

(b) [**] CAN$ ([**] Canadian Dollars) to be paid within [**] days after the commencement of the first Phase I clinical trial for any Licensed Products; and

(c) 100,000.00 CAN$ (one hundred thousand Canadian Dollars) to be paid within [**] days after the commencement of the first Phase II clinical trial for any Licensed Products; and

(d) [**] CAN$ ([**] Canadian Dollars) to be paid within [**] days after the commencement of the first Phase III clinical trial for any Licensed Products; and

(e) [**] CAN$ ([**] Canadian Dollars) to be paid within [**] days after [**]; and

(f) 200,000.00 CAN$ (two hundred thousand Canadian Dollars) to be paid within [**] days after the obtaining of the first EMEA Approval for any Licensed Products.

5.4 In addition to the milestone payments as referred to in Section 5.3 and in further consideration of the Sublicense granted hereunder, AMT shall make the following payments with respect to each additional Licensed Product, provided that said additional Licensed Product has a clinical indication different from a Licensed Product under which AMT has made payments under Section 5.3(b), 5.3(c), 5.3(d), 5.3(e) or 5.3(f):

(a) [**] CAN$ ([**] Canadian Dollars) to be paid within [**] days after the commencement of each additional Phase I clinical trial for a Licensed Product; and

(b) [**] CAN$ ([**] Canadian Dollars) to be paid within [**] days after the commencement of each additional Phase II clinical trial for a Licensed Product; and

(c) [**] CAN$ ([**] Canadian Dollars) to be paid within [**] days after the commencement of each additional Phase III clinical trial for a Licensed Product; and

(d) [**] CAN$ ([**] Canadian Dollars) to be paid within [**] days after the obtaining of each additional NDA Approval for a Licensed Product; and

(e) [**] CAN$ ([**] Canadian Dollars) to be paid within [**] days after the obtaining of each additional EMEA Approval for a Licensed Product.

5.5 In the event clinical trails for any indication will start with a combined Phase I/ Phase II trial, the milestone payment for the combined Phase I/ Phase II trial will be CAN$ 100,000.00 (one hundred thousand Canadian Dollars) and no separate Phase I or Phase II milestone payments will be due.

5.6 In the event AMT shall receive compensation from a third party relating to the Xenon Licensed Technology or to Licensed Products in any other form than in the form of a royalty, such as in the form of milestone payments, AMT shall pay to Xenon either an amount equal to [**]% ([**] percent) of the full amount of such payments to be received from the third party or the milestone payments specified in Section 5.3 and/or Section 5.4, whichever is higher.

5.7 If Xenon would play a significant role in initiating or facilitating a definitive partnership between AMT and a pharmaceutical company with respect to the development of Xenon Licensed Technology or Licensed Products, Xenon's share of the payments to be received by AMT from a third party, as referred to in Section 5.6, shall increase from [**] % to [**] % ([**] percent). If AMT and Xenon would not be able to agree on the significance of the role of Xenon as aforesaid within 30 days after the date of execution of the agreement between AMT and a pharmaceutical company, AMT and Xenon shall appoint a mutual acceptable person as an independent evaluator to determine whether or not Xenon is entitled to such increase in payment. The decision made by the evaluator will be final and binding for the Parties.

5.8 AMT will reimburse Xenon for any and all costs Xenon incurs in relation to the obtaining of [**]' consent pursuant to Section 3.2(a) of the License Agreement, provided that AMT has first provided Xenon with its written approval regarding any such Xenon expenditures, such approval which will not be unreasonably withheld.

5.9 AMT shall pay to Xenon the milestones due under Sections 5.3 and 5.4, in the timeframes provided under Sections 5.3 and 5.4, with the following exceptions:

(a) If AMT ceases all development of a particular Licensed Product (the "**First Product**") after having made payments with respect to such First Product

under Sections 5.3 and 5.4 (as applicable), following the accomplishment of any of the aforementioned milestones, there shall be no milestone payment due under Sections 5.3 and 5.4 (as applicable) upon the accomplishment of that same milestone with respect to a subsequent Licensed Product (the "**Subsequent Product**") for the same clinical indication, provided that the accomplishment of the milestone relating to the Subsequent Product occurs within [**] years following the accomplishment of the same milestone with respect to the First Product for the same clinical indication;

(b) If the First Product and the Subsequent Product, referred to in Section 5.9(a) above, are both being developed at the same time, AMT may delay paying the relevant milestones under Sections 5.3 and 5.4 (as applicable) and Section 5.9(a) as they relate to the Subsequent Product, until the earlier of:

(i) such time as AMT has ceased development of the First Product, or

(ii) until such time as the Subsequent Product receives United States New Drug Approval; and

5.10 For greater certainty, and notwithstanding any provisions within this Agreement to the contrary, the parties agree that:

(a) When milestones are achieved with respect to any Subsequent Product which were not previously paid with respect to a corresponding First Product for the same clinical indication, such milestone payments shall be paid pursuant to Sections 5.3 and/or 5.4 (as applicable); and

(b) If AMT receives United States New Drug Approval for any Licensed Product, all milestone payments due under Sections 5.3 and/or 5.4 (as applicable) shall be immediately due and owing for that particular Licensed Product, and paid forthwith at that time by AMT.

5.11 The payments as referred to in this Article 5 will not be offset against any royalties.

ARTICLE 6 - ROYALTY

6.1 In consideration of the rights and Sublicense granted hereunder by Xenon to AMT, AMT shall pay to Xenon during the Term a royalty on Net Sales, as follows:

(a) [**]% ([**] percent) on Net Sales made in any country where a Xenon Licensed Technology or Licensed Product is covered by a valid patent claim; and

(b) [**]% ([**] percent) on Net Sales made in any country where a Xenon Licensed Technology or a Licensed Product is not covered by a valid patent claim.

6.2 In the event AMT shall grant (sub)licenses for the Xenon Licensed Technology or Licensed Products to any third parties and provided that such Xenon Licensed Technology or Licensed Products are subject to protection by a valid patent claim, AMT shall make the following payments to Xenon:

(a) [**]% ([**] percent) of all royalties received from any such (sub)licensee on the sales of Xenon Licensed Technology and Licensed Products by such (sub)licensee; or

(b) [**]% ([**] percent) of all royalties received from any such (sub)licensee on the sales of Xenon Licensed Technology and Licensed Products by such (sub)licensee, in the event Xenon has played a significant role in initiating and facilitating a definitive partnership between AMT and a pharmaceutical company, as referred to in Section 5.7.

6.3 In the event AMT will be obliged to pay stacking royalties to independent third parties for the obtaining of one or more licenses to use technologies which are essential to legally market and/or sell the Licensed Products, [**].

6.4 Within [**] days following the end of each calendar quarter during the Term and within [**] days following the end of the calendar quarter in which the Term will expire, AMT shall send to Xenon a written report showing the total amount of Net Sales, specified per Xenon Licensed Technology and Licensed Product and per

country, during the preceding calendar quarter and the amount of royalties and other compensation or consideration received from its (sub)licensees, Affiliates or other third parties related to the Xenon Licensed Technology and Licensed Products.

6.5 Concurrently with sending the written report as referred to in Section 6.4, AMT shall pay to Xenon the total amount of all monies due in respect of the preceding calendar quarter by transfering such monies to a bank account designated by Xenon, in the name of Xenon, and/or (in the alternative) by an alternative method of payment, in accordance with Xenon's instructions from time to time in respect of such payments.

6.6 All amounts referred to in this Agreement are net of any source and withholding taxes. Any source or withholding taxes payable in connection with any payments made hereunder are for the account of Xenon.

ARTICLE 7 - CONTROL OF PAYMENTS

7.1 AMT shall keep at its principal place of business true, clear and separate accounts and records with respect to the Net Sales and of all other payments received from its (sub)licensees, Affiliates or other third parties and of all amounts payable to Xenon hereunder.

7.2 Xenon shall have the right to appoint a certified public auditor or accountant reasonably acceptable to AMT, which, during the regular business hours of AMT, and upon reasonable notice, may examine such accounts and records to the extent necessary only for the purpose of verifying the reports and payments required hereunder. AMT shall furnish such reasonable evidence as such representative will deem necessary to verify the accounting and will permit such representative to make copies of or extracts from such accounts, records and agreements at Xenon's expense. If an inspection of AMT records by Xenon shows an under-reporting or under payment by AMT of any amount to Xenon, in excess of [**]% for any 12 month period, then AMT shall reimburse Xenon for the cost of the inspection as well as pay to Xenon any amount found due (including any late payment charges or interest) within [**] days of notice by Xenon to AMT. All accounts and records shall be retained for [**] years from the date of their origin. Any audit under this Section 7.2 shall be at the expense of Xenon.

7.3 The calculation of royalties shall be carried out in accordance with:

(a) generally accepted Canadian accounting principles ("GAAP");

(b) the standards and principles adopted by the U.S. Financial Accounting Standards Board ("FASB"); and/or

(c) any other generally accepted and comparable accounting principle standard(s) that may be agreed upon in writing between the parties from time to time,

any or all of the above which will be applied on a consistent basis.

ARTICLE 8 - CONFIDENTIALITY, PUBLICATION

8.1 During the Term of this Agreement and for a period of ten (10) years following the termination of this Agreement, each Party shall maintain in confidence all information disclosed by the other Party which is identified as confidential and which is confirmed in writing and marked "confidential" or otherwise properly labelled within thirty (30) days of such original disclosure, including without limitations, any and all knowledge, know-how, information and/or information and/or techniques related to the Xenon Licensed Technology, Licensed Products, or the Project, and shall not, except as permitted by this Agreement, use such information or disclose the same to anyone other than those of its Affiliates, its (sub)licensees, direct employees, consultants, investigators and collaborators otherwise as is necessary in connection with such Party's activities, responsibilities and obligations as set forth in this Agreement. Each Party shall obtain a written agreement prior to disclosure to such Affiliates, (sub)licensees, direct employees, consultants or investigators containing obligations to hold in confidence and not make use of such information for any purposes other than as permitted by this Agreement, which obligations will not be less stringent than the obligations in this Section 8.2.

Each Party shall use a similar effort to that which it uses to protect its own trade secrets or proprietary information to ensure that its Affiliates, its (sub)licensees, direct employees, consultants, collaborators and investigators do not disclose or make any unauthorised use of such information.

8.2 The confidentiality obligations of Section 8.1 shall not apply to the extent that:

(a) the Party who has received the information (hereinafter referred to as "RECIPIENT") is required to disclose information by order or regulation of a governmental agency or court of competent jurisdiction or

(b) the RECIPIENT can demonstrate that:

(i) the disclosed information was at the time of such disclosure by the RECIPIENT already in the public domain other than as a result of actions of the RECIPIENT, its Affiliates, direct employees, (sub)licen-sees, consultants or investigators, in violation hereof; or

(ii) the disclosed information was rightfully and lawfully known by the RECIPIENT (as shown by its written records) prior to the date of disclosure to the RECIPIENT in connection with this Agreement or was independently developed without regard to the information; or

(iii) the disclosed information was received by the RECIPIENT (as shown by its written records) on an unrestricted basis from a source unrelated to any Party and not under a duty of confidentiality to the other Party.

8.3 Except as provided by law, each Party shall hold the existence and contents of this Agreement in strictest confidence, unless it has obtained the prior written consent thereto from the other Party, such consent not to be unreasonably withheld. Said consent is not required for the disclosure of the contents of this Agreement to UBC.

ARTICLE 9 - PATENT INFRINGEMENT

9.1 In the event that AMT or any of its sublicensees, as a result of the use of the Xenon Licensed Technology and/or any Licensed Products, is warned or sued by a third party alleging or charging infringement of any patents or patent applications known to the public after the Effective Date, AMT shall promptly notify Xenon.

9.2 Upon receiving the prior written consent of Xenon, such consent not to be unreasonably withheld, AMT shall have the right, at its expense, for settling or defending such warning or litigation for patent infringement in which the alleged infringing process or product giving rise to liability for damages includes any use of the Xenon Licensed Technology by AMT or any of its (sub)licensees. AMT acknowledges and agrees that Xenon's ability to provide such written consent may be contingent upon Xenon being able to obtain the prior consent of [**], UBC, and/or AMC, as appropriate. In such case, AMT will consult with Xenon regarding its decisions in such settlement and defence, and Xenon shall co-operate fully with AMT, so long as all the direct and indirect costs and expenses of bringing and conducting any such defence or settlement shall be borne by AMT, or its (sub)licensees, and in such event all recoveries shall enure to AMT or its (sub)licensees.

Notwithstanding the foregoing, no decision or action concerning or governing any final disposition of a warning or suit shall be taken without full consultation and approval by Xenon. Xenon and/or UBC may also elect to participate formally in any litigation involving the warning or suit to the extent that the court may permit, but in such case any additional expenses of Xenon or UBC generated by such formal participation will be paid by Xenon or UBC, as appropriate, (subject to the possibility of recovery of some or all of their respective additional expenses from the litigant).

9.3 Each of the Parties will refrain from any and all acts that may harm the patentability of the Xenon Licensed Technology in any jurisdiction.

9.4 In the event that any of the Parties is of the opinion that the Xenon Licensed Technology is or could be infringed by any third party, the Parties shall consult together on the actions to be taken against the concerning third party.

In the event the Parties will decide to take any such actions, including the institution of legal proceedings jointly, the Parties shall share the costs thereof and all compensations to which such infringing third party may be sentenced to pay.

In the event any of the Parties will decide not to participate in any action against the infringing third party, the other Party may take such action in its own name and at its own expense, in which event the Party which decided not to participate, shall render all necessary assistance to such actions.

ARTICLE 10 - REPRESENTATIONS, WARRANTIES AND COVENANTS

10.1 Xenon warrants and represents that it is fully authorised and entitled to enter into this Agreement and to grant all rights and licenses hereunder to AMT. Xenon furthermore warrants and represents that further to Section 4.1 of the License Agreement, and as evidenced by UBC's endorsement on the signatory page of this Agreement, UBC has approved Xenon's granting of the rights and licenses hereunder to AMT.

10.2 Xenon warrants and represents that it has been advised by UBC that Dr. Michael Hayden has assigned all of his intellectual property rights, if any, to the Xenon Licensed Technology to UBC.

10.3 Except as otherwise provided for in this Agreement, AMT shall indemnify, hold harmless and defend Xenon and UBC, their respective Governors, Directors, Officers, employees, faculty, students, invitees, and agents against any and all liabilities (including product liability), damages, losses or injury, death, costs, and expenses, whether direct or indirect, consequential, incidental or otherwise, including attorney's fees and associated disbursements, arising in any manner from the Collaborative Research Agreement, the License Agreement, this Agreement and/or any sub-sublicense granted by AMT under this Agreement, including the receipt or use by AMT or AMT's sub-licensees of any technology, data or other results, arising from or out of same, howsoever the same may arise.

10.4 AMT hereby covenants to observe and perform the terms and conditions contained in the License Agreement, to the extent that the same are applicable.

10.5 AMT shall use its commercially best efforts to develop, promote, market and sell all Xenon Licensed Technology and Licensed Products.

10.6 AMT covenants and agrees that it shall provide to Xenon on each of the first five anniversaries of the Commencement Date of this Agreement, a written report (the

"**Status Report**") summarizing. AMT's development activities relating to the Xenon Licensed Technology that sets out all of the following information:

(a) a summary of the research and development activities that AMT has undertaken in the course of the preceding 12 months to develop and commercialize the Xenon Licensed Technology;

(b) a detailed summary of any and all improvements, variations, updates, modifications and enhancements to the Xenon Licensed Technology which AMT has developed and/or acquired in the course of the preceding 12 months, including any improvements, variations, updates, modifications and enhancements to the Xenon Licensed Technology of which AMT has been advised by any sub-sublicensee; and

(c) any and all corporate alliances formed by AMT related to the Xenon Licensed Technology in the course of the preceding 12 months, including any such corporate alliances of which AMT has been advised by a sub-sublicensee.

10.7 Notwithstanding Section 10.1, Xenon makes no representations, conditions or warranties, either express or implied, with respect to Xenon Licensed Technology or the Licensed Products. Without limiting the generality of the foregoing, Xenon specifically disclaims any implied warranty, condition or representation that the Xenon Licensed Technology or the Licensed Products:

(a) shall correspond with a particular description;

(b) are of merchantable quality;

(c) are fit for a particular purpose; or

(d) are durable for a reasonable period of time.

Xenon shall not be liable for any loss, whether direct, consequential, incidental or special, which AMT suffers arising from any defect, error, fault or failure to perform with respect to the Xenon Licensed Technology or Licensed Products, even if AMT has been advised of the possibility of such defect, error, fault or failure. AMT

acknowledges that it has been advised by Xenon to undertake its own due diligence with respect to the Xenon Licensed Technology.

10.8 AMT will obtain, at its expense, all registrations and regulatory approvals necessary for it to exploit any of the Xenon Licensed Technology or Licensed Products. Nothing in this Agreement shall be construed as:

(a) a warranty or representation by Xenon as to title to the Xenon Licensed Technology and/or that anything made, used, sold or otherwise disposed of under the Sublicense granted in this Agreement is or will be free from infringement of patents, copyrights, trade-marks, industrial design or other intellectual property rights;

(b) an obligation by Xenon to bring or prosecute or defend actions or suits against third parties for infringement of patents, copyrights, trade-marks, industrial designs or other intellectual property or contractual rights; or

(c) the conferring by Xenon of the right to use in advertising or publicity the name Xenon or UBC or their respective trade-marks.

ARTICLE 11 - INSURANCE

11.1 Unless satisfactory arrangements are made between AMT and Xenon with respect to a self-insurance program or the requirement for insurance hereunder is waived by Xenon [**] days prior to the commencement of any human clinical trials or other Licensed Product testing involving human subjects by

AMT or any sub-sublicensee, then AMT shall procure and maintain, during the term of this Agreement, the insurance outlined in Sections 11.2 and 11.3 and otherwise comply with the insurance provisions contained in Sections 11.2 and 11.3.

11.2 AMT shall give written notice to Xenon:

(a) [**] days prior to the commencement of any human clinical trials or the Product testing involving human subjects by AMT or any sub-sublicensee (the "**Human Clinical Trials**"); and

(b) [**] days prior to the first sale of any Licensed Product by AMT or any sub-sublicensee of the terms and amount of the appropriate public liability, product liability and errors and omissions insurance which it has placed. Such insurance shall in no case be less than the insurance which a reasonable and prudent businessperson carrying on a similar line of business would acquire. This insurance shall be placed with a reputable and financially secure insurance carrier, shall include Xenon, UBC, UBC's Board of Governors, their respective directors, faculty, officers, employees, students and agents as additional insureds, and shall provide primary coverage with respect to the activities contemplated by this Agreement. Such policy shall include severability of interest and cross-liability clauses and shall provide that the policy shall not be cancelled or materially altered except upon at [**] days' written notice to Xenon. Xenon shall have the right to require reasonable amendments to the terms or the amount of coverage contained in the policy. Failing the parties agreeing on the appropriate terms or the amount of coverage, then the matter shall be determined by arbitration. AMT shall provide Xenon with certificates of insurance evidencing such coverage 20 days before commencement of Human Clinical Trials and [**] days prior to the sales of any Licensed Product and AMT covenants not to start Human Clinical Trials, or sell any Licensed Product before such certificate is provided and approved by Xenon, or to sell any Licensed Product at any time unless the insurance outlined in this Section 11.2 is in effect.

11.3 AMT shall require that each sub-sublicensee under this Agreement shall procure and maintain, during the term of the sub-sublicense, public liability, product liability and errors and omissions insurance in reasonable amounts, with a reputable and financially secure insurance carrier or provide satisfaction arrangements through an appropriate self-insurance program. AMT shall use its best efforts to ensure that any and all such policies of insurance required pursuant to this Article shall contain a waiver of subrogation against the University, Xenon, UBC's Board of Governors and their respective directors, faculty, officers, employees, students and agents.

ARTICLE 12 - TERM AND TERMINATION

12.1 All rights, obligations and responsibilities of the Parties in relationship to the Collaborative Research Agreement shall terminate upon the expiry of the Contract Period. In the event of a delay of the Project, the Parties shall consult together on the extension of the Contract Period.

12.2 This Agreement shall commence on the Effective Date, and may be terminated upon mutual written agreement between the Parties.

12.3 Each Party will have the right to terminate this Agreement, without judicial intervention, upon written notice to the other Party with immediate effect, if such other Party:

(a) passes a resolution or a Court makes an order for its winding up;

(b) has a liquidator, receiver or administrator appointed over its business or any material part of its assets;

(c) is or becomes insolvent; or

(d) ceases to carry on its business.

12.4 Unless otherwise provided under this Agreement, if either Party defaults in the performance of, or fails to be in compliance with, any condition or covenant of this Agreement and any such default or non-compliance shall not have been remedied, or steps initiated to remedy the same to the other Party's reasonable satisfaction within [**] days after receipt by the defaulting Party of a written notice thereof from the other Party, the Party not in default may forthwith terminate this Agreement at its option by giving written notice to the other Party.

12.5 Any delays in or failure of performance by Xenon under this Agreement shall not be considered a breach of this Agreement if and to the extent caused by occurrences beyond the reasonable control of Xenon, including but not limited to acts of God, regulations or laws of any government, strikes or other considered acts of workers, fires, floods, explosions, riots, wars, rebellion and sabotage, and any time for

performance hereunder shall be extended by the actual time of delay caused by such occurrence, or caused by any other circumstances which were unknown or unforeseen at the Effective Date.

12.6 Termination of this Agreement by any Party for any reason shall not affect the rights and obligations of the Parties accessed prior to the effective date of termination of this Agreement. No termination or expiry of this Agreement, however effectuated, shall release the Parties from their rights and obligations under Section 2.1(j), Article 7, Article 8, and Article 10 which shall survive the termination or expiration of this Agreement.

ARTICLE 13 - INVALIDITY, UNENFORCEABILITY

13.1 If any provision(s) of this Agreement are or become invalid, or 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. 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 which shall be valid, legal, and enforceable, and shall be mutually agreed by the Parties.

ARTICLE 14 - ENTIRE AGREEMENT, AMENDMENTS

14.1 This Agreement, including all Annexes attached hereto, contains the entire agreement of the Parties regarding the subject matter hereof and supersedes all prior agreements, understandings and negotiations regarding the same, and further, also supersedes the Heads of Agreement entered into between the Parties on August 3, 2000. This Agreement may not be changed, modified, amended or supplemented except by a written instrument signed by both Parties hereto.

ARTICLE 15 - NOTICES

15.1 All notices required by this Agreement shall be in writing. All notices and reports shall be sent by telefax or mailed by airmail, postage prepaid to the Parties at the

following addresses or such other addresses as may be designated in writing by the respective Parties:

To AMT:

Meibergdreef 61
1105 BA
Amsterdam, the Netherlands
Attention: Dr. Eric van der Aa

To Xenon:

Suite 100 — 2386 East Mall
Vancouver, B.C.
Canada V6T 1Z3
Attention: Dr. Shafique Fidai

Any notices shall be deemed given when received by the other Party.

ARTICLE 16 - RELATIONSHIP

16.1 Nothing contained in this Agreement is intended nor is to be construed so as to constitute AMT and Xenon as partners or joint venturers with respect to this Agreement. Neither Party hereto shall have any express or, implied right or authority to assume or create any obligations on behalf of or in the name of the other Party or to bind the other Party to any other contract, agreement or undertaking with any third party.

ARTICLE 17 - WAIVER

17.1 The waiver by either Party of a breach of any provisions contained herein shall be in writing and shall in no way be construed as a waiver of any succeeding breach of such provision or the waiver of the provision itself.

ARTICLE 18 - GOVERNING LAW, DISPUTES

18.1 This Agreement shall be construed and interpreted according to the laws of the Province of British Columbia and the laws of Canada in force therein without regard to its conflict of law rules.

18.2 Any disputes arising out of or in relation to this Agreement shall, to the exclusion of all others, be referred to the Supreme Court of British Columbia.

ARTICLE 19 - GENERAL

19.1 Time shall be of the essence of this Agreement.

21.2 The Parties acknowledge and agree that the *International Sale of Goods Contracts Convention Act* and the United Nations Convention on Contracts for the International Sale of Goods have no application to this Agreement.

21.3 This Agreement may be executed in counterparts, or facsimile counterparts, each of which when executed by either of the Parties shall be deemed to be an original and such counterparts shall together constitute one and the same Agreement.

IN WITNESS WHEREOF, duly authorized signatories of the Parties hereto have executed this Agreement as of the date(s) indicated below, but effective as of the Effective Date.

Xenon Genetics Inc.

By:

Date: _____

Amsterdam Molecular Therapeutics B.V.

_____

By:

Date: _____

ANNEX 1 - COLLABORATIVE RESEARCH AGREEMENT

COLLABORATIVE RESEARCH AGREEMENT

This Agreement dated for reference the 1st day of August, 2000.

BETWEEN:

XENON GENETICS INC., a Canadian corporation having an address at suite 100 - 2386 East Mall, Vancouver, BC, V6T 1Z3

(the "***Sponsor***")

AND:

THE UNIVERSITY OF BRITISH COLUMBIA, a corporation continued under the *University Act* of British Columbia and having its administrative offices at 2075 Wesbrook Mall, in the City of Vancouver, in the Province of British Columbia, V6T 1W5;

(the *"**Research Organization**"*)

WHEREAS:

A. The research program contemplated by this Agreement is of mutual interest and benefit to the Research Organization and to the Sponsor, will further the instructional and research objectives of the Research Organization in a manner consistent with its status as a non-profit, tax-exempt, educational institution, and may derive benefits for both Sponsor and Research Organization through inventions, improvements, and/or discoveries; and

B. The parties acknowledge that Dr. Michael Hayden has an appointment within the Research Organization and is also Chief Scientific Officer of the Sponsor and that Dr. Michael Hayden will be required to comply with the policies of the Research Organization relating to conflicts of interest.

NOW THEREFORE THIS AGREEMENT WITNESSETH that in consideration of the premises and of the mutual covenants herein set forth, the parties hereto have covenanted and agreed as follows:

1.0 DEFINITIONS:

1.1 In this Agreement, unless a contrary intention appears, the following words and phrases shall mean:

(a) *"**Project**"* shall mean the project as specifically described in Appendix "**A**" hereof which shall be carried out under the direction of the Investigator.

(b) *"**Investigator**"* shall mean Dr. Michael Hayden of the Department of Medical Genetics at the University of British Columbia.

(c) *"**Contract Period**"* shall mean August 1, 2000 through July 31, 2002.

(d) *"**Confidential Information**"* shall mean any and all knowledge, know-how, information, and/or techniques disclosed by the one party (referred to in this capacity as the *"**Provider**")* to another (referred to in this capacity as the *"**Recipient**")* relating to the Project, including, without limiting the generality of the foregoing, all research, data, specifications, plans, drawings, prototypes, models, documents, records, instructions, manuals, papers, or other materials of any nature whatsoever, whether written or otherwise, relating to same. In order to constitute *"Confidential Information"* for the purposes of this Agreement, the Provider must clearly identify it in writing as being confidential, or if the disclosure takes place orally or in some other non-tangible form, the Provider must summarize it in writing and identify it as being confidential within 30 days of making the disclosure. Furthermore, such disclosures shall not be considered *"Confidential Information"* for the purposes of this Agreement if and when it:

(i) is made subject to an order by judicial or administrative process requiring the Recipient to disclose any or all of the Confidential Information disclosed to it by the Provider, provided however that the Recipient shall promptly notify the Provider and allow the Provider reasonable time to oppose such process before disclosing any of the Confidential Information disclosed to it by the Provider;

(ii) is published or becomes available to the general public other than through a breach of this Agreement;

(iii) is obtained by the Recipient from a third party with a valid right to disclose it, provided that said third party is not under a confidentiality obligation to the Discloser;

(iv) is independently developed by employees, agents or consultants of the Recipient who had no knowledge of or access to the Confidential Information disclosed to it by another party to this Agreement as evidenced by the Recipient's business records; or

(v) was possessed by the Recipient prior to receipt from the Provider, other than through prior disclosure by the Provider, as evidenced by the Recipient's business records.

(e) *"**License Agreement**"* shall mean the License Agreement dated as of August 1, 2000 between the Sponsor and the Research Organization.

(f) *"**Research Organization Intellectual Property**"* shall mean, individually and collectively, all inventions, improvements, and/or discoveries which are conceived and/or made by one or more employees of the Research Organization during the Contract Period in the performance of the Project.

(g) *"**Sponsor Intellectual Property**"* shall mean, individually and collectively, all inventions, improvements, and/or discoveries which are conceived and/or made solely by one or more employees of the Sponsor during the Contract Period in the performance of the Project.

(h) *"**Joint Intellectual Property**"* shall mean, individually and collectively, all inventions, improvements, and/or discoveries which are conceived and/or made jointly by one or more employees of the Research Organization and by one or more employees of the Sponsor and/or any of the Sponsor's sublicensees under the License Agreement during the Contract Period in the performance of the Project.

2.0 RESEARCH WORK:

2.1 The Research Organization shall commence the performance of the Project promptly after the effective date of this Agreement, and shall use reasonable efforts to perform the Project substantially in accordance with the terms and conditions of this Agreement. Notwithstanding anything to the contrary in this Agreement, the Sponsor and the Research Organization may at any time amend the Project by mutual written agreement.

2.2 In the event that the Investigator becomes unable or unwilling to continue the Project, and a mutually acceptable substitute is not available, the Research Organization and the Sponsor shall each have the option to terminate the Project and this Agreement by providing the other party with written notice of same.

2.3 In the performance of all services hereunder:

(a) the Research Organization shall be deemed to be and shall be an independent contractor;

(b) neither party is authorized or empowered to act as agent for the other for any purpose and shall not on behalf of the other enter into any contract, warranty, or representation as to any matter; and

(c) neither party shall be bound with respect to third parties by the acts or conduct of the other.

3.0 REPORTS AND CONFERENCES:

3.1 Interim written project reports shall be provided by the Research Organization to the Sponsor from time to time during the Contract Period, at mutually agreed to intervals, and a final project report shall be submitted by the Research Organization to the Sponsor within 60 days after the conclusion of the Contract Period or early termination of this Agreement, whichever is sooner.

3.2 Interim written financial statements shall be provided by the Research Organization to the Sponsor from time to time during the Contract Period, at mutually agreed to intervals, and a final financial statement shall be submitted by the Research Organization to the Sponsor within 60 days after the conclusion of the Contract Period or early termination of this Agreement, whichever is sooner.

3.3 During the term of this Agreement, representatives of the Research Organization will meet with representatives of the Sponsor at times and places mutually agreed upon to discuss the progress and results, as well as ongoing plans, or changes therein, of the Project.

3.4 The Project will be managed and co-ordinated by a Research Steering Committee, which shall be authorised to take all decisions in respect of the Project, as follows:

(a) The Research Steering Committee will consist of two representatives on behalf of the Sponsor and two representatives on behalf of the Research Organization, all representatives each having one vote. A representative on behalf of the Sponsor will be the chair of the Research Steering Committee;

(b) All Project decisions will be taken unanimously. If the votes are equally divided, the issue voted on will be submitted to advisors appointed by the Research Organization and the Sponsor (presently [**] on behalf of the Sponsor and [**] on behalf of the Research Organization). If the two advisors are not be able to reach an agreement on the issue concerned, the issue will be discussed by the Director, University Industry Liaison Office of the Research Organization and the President/CEO of the Sponsor in order to try to reach an agreement. If an agreement cannot be reached in a timely fashion by these two individuals, the arbitration provisions of Article 14.2 will then apply;

(c) Upon request of the Sponsor, the representative of the Research Organization who will be responsible for the execution of the research activities under the Project within the Research Organization will attend the meeting of the Research Steering Committee and shall give all necessary information on the execution of the Project that the Sponsor and/or the Research Organization may desire;

(d) The Research Steering Committee shall meet either by telephone, videoconference or in person either in Vancouver or in Amsterdam. Minutes of all meetings will be made by a secretary to be appointed by the Sponsor, who will attend all meetings. The Sponsor will send minutes of each meeting to all members of the Research Steering Committee at least two (2) weeks before the date of the next meeting; and

(e) If the parties agree to hold the meeting in Amsterdam all reasonable travel costs to be incurred by the representatives of the Sponsor and/or the Research Organization in the Research Steering Committee and if so requested by the Sponsor, any other employees of the Sponsor or the Project leader of the Research Organization, will be paid in advance where possible, and/or reimbursed by the Sponsor, provided that the Sponsor has given its prior consent to each travel plan and the approximate cost thereof.

3.5 All employees of the Research Organization working on the Project shall, on a day to day basis, keep written records of all research activities performed and Research Organization Intellectual Property and Joint Intellectual Property conceived and/or made during the performance of the Project, according to commonly accepted standards of workmanship. In any event, all records shall be of such quality and meet standards that reasonably comply with generally accepted pre-clinical requirements.

3.6 The Research Organization shall devote reasonable efforts (having regard to the constraints and working environment of an academic laboratory) to provide the Sponsor with all study protocols and final reports of all individual studies with respect to the Project in the Sponsor's format and according to the Sponsor's standards within 30 (thirty) days after the end of each calendar quarter.

3.7 At the Sponsor's discretion, all rights and responsibilities of the Sponsor above may be performed by the Sponsor's designate, or approved sublicensee or assignee, as appropriate.

4.0 COSTS, BILLINGS, AND OTHER SUPPORT:

4.1 It is agreed to and understood by all parties that, subject to Article 4.3 and 12, and notwithstanding anything else to the contrary in this Agreement, the total costs to the Sponsor relating to the Project hereunder shall be no greater than $600,000. The parties acknowledge that any budget categories that may be set forth in the description of the Project are estimates only and that changes from category to category may be made at the Research Organization's discretion. No invoice shall be issued to the Sponsor by the Research Organization. Payment in advance shall be made by the Sponsor according to the following schedule:

Payment Due Date		Amount Due
On execution of this Agreement	$	75,000.00
December 29, 2000	$	75,000.00
March 30, 2001	$	75,000.00
June 29, 2001	$	75,000.00
September 28, 2001	$	75,000.00
December 31, 2001	$	75,000.00
March 28, 2002	$	75,000.00
June 28, 2002	$	75,000.00
Total:	**$**	**600,000.00**

The Research Organization reserves the right to suspend work on the Project or to terminate the Project and this Agreement by delivering written notice of same to the Sponsor if the Sponsor fails to make the aforementioned payments within 30 days of the dates herein specified.

4.2 The Research Organization shall retain title to any equipment purchased with funds provided by the Sponsor under this Agreement.

4.3 In the event of early termination of this Agreement, the Sponsor shall pay all costs and liabilities relating to the Project which have been incurred by the Research Organization as of the date of receipt of notice of such termination. For greater certainty, such costs and liabilities shall include all non-cancellable obligations including payments in lieu of reasonable notice for technicians, graduate students, and other staff assigned to the Project.

5.0 PUBLICITY:

5.1 Notwithstanding anything to the contrary in this Agreement, the Research Organization may disclose the identity of the Sponsor, the title of the Project, the name of the Investigator, the Contract Period, and the amount of funding being provided by the Sponsor in support of the Project. Further, the Sponsor and any sublicensee thereof may (subject to the confidentiality provisions of Article 6) disclose the existence and nature of this Agreement, the amount of funding being provided and the nature of the Project without the need for the consent of the Research Organization. Except as provided by the foregoing, neither party may use the name of the other, nor of any member of the other's Project staff, in any publicity, advertising, or

news release, unless required to do so by law, without the prior written approval of an authorized representative of the other, such approval not to be unreasonably withheld.

6.0 CONFIDENTIALITY:

6.1 The Recipient shall not use the Confidential Information provided to it by the Provider, directly or indirectly, for any purpose other than as specifically set forth in this Agreement. Without limiting the generality of the foregoing, the Recipient shall not use, manufacture, or sell the Provider's Confidential Information or any device or means incorporating any of the Provider's Confidential Information, and shall not use any of the Provider's Confidential Information as the basis for the design or creation of any device or means.

6.2 The Recipient shall keep and use all of the Provider's Confidential Information in confidence and shall not disclose any part of the Provider's Confidential Information to any person, firm, corporation, or other entity with the exception that the Sponsor may disclose Confidential Information provided to it from the Research Organization to the Sponsor's sublicensees under the License Agreement on terms consistent with the confidentiality restrictions set forth in this Collaborative Research Agreement and the License Agreement. In the event that there is any inconsistency between the confidentiality provisions in this Agreement and those provided for in the License Agreement, the terms of this Agreement will prevail with respect to any Confidential Information under the terms of this Agreement.

6.3 Subject to Article 5.1, the Sponsor requires of the Research Organization, and the Research Organization agrees insofar as it may be permitted to do so at law, that this Agreement, and each part of it, is confidential and shall not be disclosed to third parties, as the Sponsor claims that such disclosure would or could reveal commercial, scientific or technical information and would significantly harm the Sponsor's competitive position.

6.4 Notwithstanding any termination or expiration of this Agreement, the obligations of confidentiality set forth in this Article 6 shall survive and continue to be binding upon the Recipient, its successors, and assigns until three (3) years after such termination or expiration.

7.0 PUBLICATIONS:

7.1 The Sponsor acknowledges that the policies of the Research Organization require that the results of the Project be publishable, subject to Article 6. The parties therefore agree that researchers engaged in the Project shall not be restricted from presenting at symposia, national, or regional professional meetings, or from publishing in abstracts, journals, theses, or dissertations, or otherwise, whether in printed or in electronic media, methods and results of the Project, provided however that:

(a) the Research Organization provides the Sponsor with copies of any proposed publication or presentation at least 45 days in advance of the submission of such proposed publication or presentation to a journal, editor, or other third party; and

(b) the Sponsor has not, within 30 days after receipt of said copies, objected in writing to such proposed presentation or proposed publication in accordance with Article 7.2 of this Agreement.

7.2 The Sponsor may object to a proposed presentation or proposed publication on the grounds that it contains Confidential Information that was disclosed to the Research Organization by the Sponsor or on the grounds that it discloses patentable subject matter which needs protection. In the event that the Sponsor makes such objection on the former ground, the Research Organization shall ensure that its researchers remove such Confidential Information immediately from the proposed presentation or publication, after which the Research Organization and its researchers may proceed with said presentation or publication. In the event that the Sponsor makes such an objection on the latter ground, it shall be deemed to be a direction to the Research Organization to file a patent application pursuant to Article 8.4, and the Research Organization shall ensure that its researchers refrain from making such publication or presentation until Research Organization has filed one or more patent applications with one or more patent offices directed to such patentable subject matter, or until 3 months have elapsed from date of receipt of such written objection from the Sponsor by the Research Organization, whichever is sooner, after which the Research Organization and its researchers may proceed with said presentation or publication. For greater certainty, a provisional patent application shall be considered to be a patent application in the United States of America for the purposes of this Agreement.

8.0 INTELLECTUAL PROPERTY:

8.1 The parties acknowledge and agree that all rights and title to Research Organization Intellectual Property shall belong to the Research Organization.

8.2 The parties acknowledge and agree that all rights and title to Sponsor Intellectual Property shall belong to the Sponsor.

8.3 Notwithstanding Article 8.1, the parties acknowledge and agree that, as between the parties, all rights and title to Joint Intellectual Property shall belong jointly to the Research Organization and the Sponsor. Notwithstanding the applicable patent or other intellectual property laws in any jurisdiction, none of the parties may commercially exploit any Joint Intellectual Property except as specifically provided for in Article 9.3.

8.4 The Research Organization will promptly notify Sponsor of any Research Organization Intellectual Property. The parties will promptly notify one another of any Joint Intellectual Property. The Sponsor may direct that one or more patent applications be filed in respect of such Research Organization Intellectual Property or Joint Intellectual Property, as the case may be, in which case the Research Organization shall promptly prepare, file, and prosecute such patent applications in such jurisdictions as the Sponsor directs in the name of the Research Organization in the case of Research Organization Intellectual Property and in the joint names of the Research Organization and the Sponsor in the case of Joint Intellectual Property. Where the parties have agreed upon a license in accordance with Article 9.2 or 9.4, as applicable, the Sponsor shall bear all costs incurred in connection with the preparation, filing, prosecution, and maintenance of such patent applications and shall cooperate with the Research Organization to assure that such patent applications cover, to the best of the Sponsor's knowledge, all items of commercial interest and importance. While the Research Organization shall be responsible for making final decisions regarding the scope and content of such patent applications and the prosecution thereof, the Sponsor shall be given an opportunity to review and provide input thereto. The Research Organization shall keep the Sponsor advised as to all developments with respect to such applications and shall promptly supply the Sponsor with copies of all papers received and filed in connection with the prosecution thereof in sufficient time for the Sponsor to comment thereon

8.5 If the Research Organization wishes to obtain patent protection in respect of Research Organization Intellectual Property and/or Joint Intellectual Property over and above that for which the Sponsor wishes to provide its financial support pursuant to Article 8.4, the Research Organization shall be free to file or continue prosecution or maintain any such applications and to maintain any protection issuing thereon at its own expense.

9.0 GRANT OF RIGHTS:

9.1 The Research Organization grants the Sponsor (subject to the rights and consent, as and if applicable, of the Academic Hospital at the University of Amsterdam, Amsterdam Molecular Therapeutics B.V., and/or [**]) the option to obtain an exclusive royalty-bearing world-wide license to use or otherwise

exploit the Research Organization's rights to any Research Organization Intellectual Property subject to terms and conditions determined in accordance with Article 9.2. Said option shall subsist with respect to each item of Research Organization Intellectual Property for a period of 6 months after said item has been disclosed in writing by the Research Organization to the Sponsor and may be exercised within this period by the Sponsor delivering written notice of same to the Research Organization.

9.2 If the Sponsor exercises its option pursuant to Article 9.1, the parties shall negotiate in good faith to determine the specific terms and conditions on which the license shall be granted by the Research Organization (and the Academic Hospital at the University of Amsterdam, Amsterdam Molecular B.V., and/or [**], as applicable and subject to their consent) to the Sponsor according to the following terms and ranges: License fee in the range of [**] and royalties in the range of either (i) [**] or (ii [**]. If the parties are unable to agree upon such specific terms and conditions within a period of 6 months after the date when the Sponsor exercised its option, the Sponsor shall have the right to have the terms and conditions which are still in issue determined by an arbitrator in accordance with Article 14. Said arbitrator shall be required to determine such outstanding terms and conditions:

(a) Subject to this Article 9.2, in accordance with generally accepted industry standards where such terms and conditions relate purely to financial matters such as minimum annual royalty amounts, percentage royalty rates, and performance requirements; and

(b) Substantially in accordance with the then current licensing practices of the Research Organization in all other matters including, without limiting the generality of the foregoing, matters of indemnification, insurance, confidentiality, use of trade-marks or names of Research Organization personnel, and disclaimer of warranty.

9.3 Each of the parties hereto grants the other the option to obtain a royalty-bearing license to use or otherwise exploit Joint Intellectual Property subject to terms and conditions determined in accordance with Article 9.4. Said option shall subsist with respect to each item of Joint Intellectual Property for a period of 6 months after said item has been disclosed in writing by one party to the other and may be exercised within this period by the exercising party delivering written notice of same to the other.

9.4 If either one of the parties exercises its option pursuant to Article 9.3, the parties shall negotiate in good faith to determine the specific terms and conditions on which the license shall be granted to the exercising party according to the terms and ranges set forth in Article 9.2. If the parties are unable to agree upon such specific terms and conditions within a period of

6 months after the date when the option was exercised, either party shall have the right to have the terms and conditions which are still in issue determined by an arbitrator in accordance with Article 14. Said arbitrator shall be required to determine such outstanding terms and conditions:

(a) Subject to this Article 9.4, in accordance with generally accepted industry standards where such terms and conditions relate purely to financial matters such as minimum annual royalty amounts, and performance requirements except that the applicable royalty rate to be paid by the exercising party to the other shall be determined by ascertaining what would be a fair market royalty rate if the applicable Joint Intellectual Property was owned in its entirety by one party and was being licensed to an independent, arms-length, third party and then dividing this figure equally by the number of joint owners of the applicable Joint Intellectual Property; and

(b) Substantially in accordance with the then current licensing practices of the Research Organization in all other matters including, without limiting the generality of the foregoing, matters of indemnification, insurance, confidentiality, use of trade-marks or names of Research Organization personnel, and disclaimer of warranty.

9.5 Subject to the Research Organization's compliance with Article 6, the Sponsor hereby grants the Research Organization a non-exclusive, royalty-free license in perpetuity to use Sponsor Intellectual Property and Joint Intellectual Property but only for academic and research purposes, and not for any commercial purposes whatsoever. Except as expressly provided for above, the Research Organization may not use or license any Joint Intellectual Property without the express prior written consent of the Sponsor.

9.6 Notwithstanding Articles 6, 9.1 and 9.3 of this Agreement, the Sponsor (and the Sponsor's sublicensees or sub-sublicensees, as the case may be) shall have access to, and the right to use, non-patentable research data and reports generated during the performance of the Project, for the limited purpose of fulfilling regulatory requirements in the research and development process relating to the technology that has been licensed to the Sponsor under the License Agreement.

10.0 <u>TERM AND TERMINATION:</u>

10.1 This Agreement shall be deemed to have come into force upon the beginning of the Contract Period and shall continue in effect for the full duration of the Contract Period unless sooner terminated in accordance with the provisions of this Article. The parties hereto may, however, extend the term of this Agreement for additional periods as desired under mutually agreeable terms and conditions which the parties reduce to writing and sign. Either party may terminate this agreement upon ninety (90) days prior written notice to the other.

10.2 In the event that either party hereto shall commit any breach of or default in any of the terms or conditions of this Agreement, and also shall fail to remedy such default or breach within thirty (30) days after receipt of written notice thereof from the other party hereto, the party giving notice may, at its option and in addition to any other remedies which it may have at law or in equity, terminate this Agreement by sending notice of termination in writing to the other party to such effect and such termination shall be effective as of the date of the receipt of such notice.

10.3 Termination of this Agreement by either party for any reason shall not affect the rights and obligations of the parties accrued prior to the effective date of termination of this Agreement pursuant to Articles 8 and 9. No, termination of this Agreement, however effectuated, shall release the parties hereto from their rights and obligations under Articles 4.3, 5, 6, 10.4, or 12.

10.4 Forthwith upon the termination of this Agreement, the Recipient shall cease to use the Provider's Confidential Information in any manner whatsoever and upon the written request of the Provider shall forthwith deliver up to the Provider all of the Provider's Confidential Information in the Recipient's possession or control, together with a certificate certifying that no copies have been made or retained.

11.0 DISCLAIMER OF WARRANTY:

11.1 The Research Organization makes no representations or warranties, either express or implied, with respect to the data or other results arising from the Project or with respect to any Confidential Information it may disclose to the Sponsor. The Research Organization specifically disclaims any implied warranty of non-infringement or merchantability or fitness for a particular purpose and shall in no event be liable for any loss of profits, be they direct, consequential, incidental, or special or other similar or like damages arising from any defect, error or failure to perform, even if the Research Organization has been advised of the possibility of such damages. The Sponsor hereby acknowledges that the Project is of an experimental and exploratory nature, that no particular results can be guaranteed, and that it has been advised by the Research Organization to undertake its own due diligence with respect to all matters arising from this Agreement.

12.0 INDEMNITY:

12.1 The Sponsor hereby indemnifies, holds harmless and defends the Research Organization, its Board of Governors, directors, officers, employees, faculty, students, invitees, and agents against any and all claims (including all legal fees and disbursements incurred in association therewith) arising out of the receipt or use by the Sponsor of any of the Research Organization's Confidential Information, the Research Organization Intellectual Property, the Joint Intellectual Property, the Sponsor Intellectual Property, or any data or other results arising from the Project including, without limiting the generality of the foregoing, any damages or losses, consequential or otherwise, arising from or out of same, howsoever the same may arise.

13.0 INSURANCE:

13.1 The parties acknowledge that the Research Organization has adequate liability insurance applicable to its officers, employees, and agents while acting within the scope of their employment by the Research Organization, and that the Research Organization has no liability insurance policy as such that can extend protection to any other person. Therefore, subject to Article 12, each party hereby assumes any risks of personal injury and property damage attributable to the negligent acts or omissions of that party and its officers, employees, and agents.

14.0 GOVERNING LAW AND ARBITRATION:

14.1 This Agreement shall be governed by and construed in accordance with the laws of the Province of British Columbia and the laws of Canada in force therein without regard to its

conflict of law rules. All parties agree that by executing this Agreement they have attomed to the jurisdiction of the Supreme Court of British Columbia. Subject to Article 14.2, the Supreme Court of British Columbia shall have exclusive jurisdiction over this Agreement.

14.2 In the event of any dispute arising between the parties concerning this Agreement, its enforceability, or its interpretation, said dispute shall be settled by a single arbitrator appointed pursuant to the provisions of the *International Commercial Arbitration Act* of British Columbia, or any successor legislation then in force. The place of arbitration shall be Vancouver, British Columbia, Canada and the language to be used in the arbitration proceedings shall be English. Notwithstanding the foregoing, either party may apply to a court of competent jurisdiction for interim protection such as, by way of example, an interim injunction.

15.0 ASSIGNMENT:

15.1 Except as specifically provided by Article 15.2, this Agreement shall not be assigned by any party without the prior written consent of the others, which consent shall not be unreasonably withheld.

15.2 The Sponsor may assign its rights and obligations pursuant to this Agreement to any majority stockholder of the Sponsor and/or any subsidiary in which the Sponsor is a majority stockholder and/or to a purchaser of all or substantially all of the assets of the Sponsor, provided that the Sponsor notifies the Research Organization in writing in advance of such assignment and further provided that the assignee enter into a written agreement with the Research Organization pursuant to which the assignee agrees to assume responsibility for all of the Sponsor's obligations pursuant to this Agreement.

16.0 NOTICES:

16.1 All notices or other documents that either of the parties hereto are required or may desire to deliver to the other party hereto may be delivered only by personal delivery or by registered or certified mail, or fax, all postage and other charges prepaid, at the address for such party set forth below or at such other address as that party may hereinafter designate in writing to the other. Any notice personally delivered or sent by fax shall be deemed to have been given or received at the time of delivery, or transmission.

Sponsor: President
Xenon Genetics Inc.
100 - 2386 East Mall
Vancouver, British Columbia
V6T 1Z3
Telephone: (604) 221-8478
Fax: (604) 221-8423

Research Organization: The Director
The University of British Columbia
University - Industry Liaison Office
I.R.C. Room 3312194 Health Sciences Mall
Vancouver, British Columbia
V6T 1Z3
Telephone: (604) 822-8580
Fax: (604) 822-8589

16.2 Questions or queries of a scientific nature or regarding financial matters may be directed by the Sponsor to the Research Organization through the following contacts:

Technical Matters: Dr. Michael Hayden
Department of Medical Genetics
The University of British Columbia
Center for Molecular Medicine and Therapeutics
Vancouver, British Columbia
Telephone:(604) 875-3535
Telecopier:(604) 875-3819

Financial Matters: Ms. Claudia Nadalini
Office of Financial Services
University of British Columbia
General Services Administration Building
2075 Wesbrook Mall
Vancouver, British Columbia
V6T 1Z1
Telephone:(604) 822-2321
Telecopier:(604) 822-2417

17.0 NO CONSULTING

17.1 The parties hereto acknowledge that, Dr. Michael Hayden *("**Dr. Hayden**")* has an appointment within the Research Organization and is also Chief Scientific Officer of the Sponsor and that Dr. Michael Hayden will be required to comply with the policies of the Research Organization relating to conflicts of interest. Without limiting the generality of the foregoing, the Sponsor and Dr. Hayden acknowledge and agree that Dr. Hayden has been engaged by the Research Organization as the Investigator in connection with the Project described in Appendix *"**A**"* to this Agreement, and that Dr. Hayden will not be engaged or retained as a consultant by the Sponsor or any other party in connection with the Project or research related to the Project.

18.0 GENERAL:

18.1 The appendices to this Agreement together with the terms and conditions contained within this Agreement constitute the entire understanding between the parties hereto and no modifications hereof shall be binding unless executed in writing by the parties hereto. The appendices will be binding upon the parties hereto except to the extent that they may conflict with the terms and conditions contained within this Agreement itself, in which case the terms and conditions of this Agreement shall govern.

18.2 In the event that any part, section, clause, paragraph or subparagraph of this Agreement shall be held to be indefinite, invalid, illegal or otherwise voidable or unenforceable, the entire agreement shall not fail on account thereof, and the balance of the Agreement shall continue in full force and effect.

18.3 No condoning, excusing or overlooking by either party of any default or breach by the other party in respect of any terms of this Agreement shall operate as a waiver of such party's rights under this Agreement in respect of any continuing or subsequent default or breach, and no waiver shall be inferred from or implied by anything done or omitted by such party, save only an express waiver in writing.

18.4 No exercise of a specific right or remedy by any party precludes it from or prejudices it in exercising another right or pursuing another remedy or maintaining an action to which it may otherwise be entitled either at law or in equity.

18.5 In the event of a conflict arising between the interpretation of this Agreement and the License Agreement, the terms of the License Agreement will prevail.

IN WITNESS WHEREOF the parties hereto have hereunto executed this Agreement effective as of the beginning of the Contract Period, regardless of the date of execution.

Signed for and on behalf of)
XENON GENETICS INC.)
by its authorized signatories:)
)
)
_____)
Authorized Signatory)
)
)
_____)
Authorized Signatory)

Signed for and on behalf of)
THE UNIVERSITY OF BRITISH COLUMBIA)
by its authorized signatories:)
)
)
_____)
Authorized Signatory)
)
)
_____)

Authorized Signatory)

I have read and understood the foregoing Agreement and understand my responsibilities as the Investigator, and in particular acknowledge and agree with the restrictions on my ability to be retained or engaged as a consultant as set out in Article 17.1 herein:

Name: Dr. Michael Hayden
Department: UBC Medical Genetics Department

APPENDIX "A"
STATEMENT OF WORK
PROJECT DESCRIPTION

LPL Gene Therapy for LPL Deficiency

1. PROJECT OBJECTIVE

AMT is developing an adeno-associated virus (MV) gene therapy strategy as an orphan drug to enhance triglyceride metabolism in genetically deficient LPL patients. When successful, this gene therapy approach has the potential to be used in large patient populations by correcting the lipoprotein phenotype in coronary artery disease and myocardial ischemia. This project is directed at the development of an adeno-associated virus (AAV), gene-based strategy for the replacement and/or enhancement of Lipoprotein Lipase (LPL) gene function in humans. The specific objectives of this gene targeting approach is to enhance intravascular triglyceride (Tg) metabolism (or in other words, lipid breakdown) in patients who have severely elevated blood Tg's due to a genetic deficiency of LPL; these patient carry a high health risk of both morbidity and mortality, and occur in significant numbers in Western Europe, Canada, the US and South Africa.

2. PROJECT DESCRIPTION

2.1. Preclinical proof of principle studies

Objectives:
- Generate and test MV vector expressing LPL
- Demonstrating proof of principle
- Evaluate gene expression

For the proof of principle study, [**].

Study design

[**]

.

Anticipated time schedule
1. [**] study
   a. Start experiment: [**]
   b. End of experiment: [**]

2. [**] study
   a. Start experiment: [**]
   b. End of experiment: [**]

Committed number of FTE's fully dedicated to this project [**]

2.2. Preclinical [**]studies

Objectives:
- Evaluate safety
- Evaluate immune response
- Evaluate gene expression
- Evaluate delivery site
- Evaluate distribution of vector to various tissues (biodistribution)
- Selection of product candidate for clinical development
- Identify doses for clinical trials
- Filing of regulatory submission to commence clinical trials
- Obtain regulatory approval to start clinical trial

[**].

Anticipated time schedule
Start: [**]

Budget

	Annual Budget [**] years	University Overhead @ [**]%"
Salaries, Equipment and Consumables		
Project Leader	$ [**]	$ [**]
Benefits [**]	[**]	[**]
Post Doctoral Fellow	[**]	[**]
Benefits [**]	[**]	[**]
[**]	[**]	[**]
Benefits [**]	[**]	[**]
Reagents and routines (consumables)($[**]/ person / month)	[**]	[**]
Minor Equipment	[**]	—
Costs for animal experiments		
Mice study *: [**]	[**]	[**]
Cat study **: ([**])	[**]	[**]
Subtotal	[**]	[**]
"TOTAL ANNUAL BUDGET:	[**]	

* [**].

** [**].

ANNEX 2 — LICENSE AGREEMENT (Redacted Form)

Annex 2

LICENSE AGREEMENT

BETWEEN:

THE UNIVERSITY OF BRITISH COLUMBIA, a corporation continued under the *University Act* of British Columbia and having its administrative offices at 2075 Wesbrook Mall, in the City of Vancouver, in the Province of British Columbia, V6T 1W5

(the *"**University**")*

AND:

XENON GENETICS INC., a corporation continued under the laws of Canada, and having its administrative offices at Suite 100 - 2386 East Mall, Vancouver, British Columbia, V6T 1Z3

(the *"**Licensee**"*)

WHEREAS:

A. The University has been engaged in research during the course of which it has invented, developed and/or acquired certain technology identified in UBC Invention Disclosure File #UBC 94-061, entitled *"Lipofipase Mutation 291, Implication for Coronary Artery Disease"*, and File #UBC 91-003, entitled *"Mutation in Human Lipoprotein Lipase Gene which causes Type 1 Hyperlipoproteinemia";*

B. [**] has invented, developed and/or acquired certain technology which may have common subject matter with certain technology invented, developed and/or acquired by the University, and identified in UBC Invention Disclosure File # UBC 99-082, entitled *"Recombinant Viruses Preparation and use thereof in Gene Therapy";*

C. The University has been jointly engaged in research with the Academic Hospital at the University of Amsterdam *("**AMC**")* during the course of which they have jointly invented, developed and/or acquired certain technology identified in UBC Invention Disclosure File # UBC 00039, entitled *"Mutation 447";*

D. The research done at the University with respect to the above referenced technologies was undertaken by Dr. Michael Hayden who is an employee of the University engaged in a number of research projects and a founder and chief scientific officer of the Licensee;

E. [**].

F. The University is desirous of entering into this agreement (the *"**Agreement**") with* the objective of furthering society's use of its advanced technology, and to generate further research in a manner consistent with its status as a non-profit, tax exempt educational institution; and

G. Subject to the terms and conditions hereinafter set out, the Licensee is desirous of the University granting a license to the Licensee to use or cause to be used the University's interest in such technology to manufacture, distribute, market, sell and/or license or sublicense products and services derived or developed from such technology.

NOW THEREFORE THIS AGREEMENT WITNESSETH that in consideration of the premises and of the mutual covenants herein set forth, the parties hereto have covenanted and agreed as follows:

1.0 DEFINITIONS:

1.1. In this Agreement, unless a contrary intention appears, the following words and phrases shall mean:

(a) *"**Accounting**":* an accounting statement setting out in detail how the amount of Revenue was determined;

(b) *"**Affiliated Company**"* or *"**Affiliated Companies**":* two or more corporations where the relationship between them is one in which one of them is a subsidiary of the other, or both are subsidiaries of the same corporation, or fifty percent (50%) or more of the voting shares of each of them is owned or controlled by the same person, corporation or other legal entity;

(c) *"**Collaborative Research Agreement**":* the Collaborative Research Agreement dated August 1, 2000 between the University and the Licensee, which contemplates the performance of a research project entitled *"LPL* Gene *Therapy for LPL Deficiency"*,

(d) *"**Confidential Information**":* any part of the Information which is designated by either party (the *"**Disclosing Party**")* as confidential, whether orally or in writing but excluding any part of the Information:

(i) possessed by the receiving party prior to receipt from the Disclosing Party, other than through prior disclosure by the Disclosing Party, as evidenced by the receiving party's business records;

(ii) published or available to the general public otherwise than through a breach of this Agreement;

(iii) obtained by the receiving party from a third party with a valid right to disclose it, provided that said third party is not under a confidentiality obligation to the Disclosing Party; or

(iv) independently developed by employees, agents or consultants of the receiving party who had no knowledge of or access to the Disclosing Party's Information as evidenced by the receiving party's business records;

(e) *"**Date of Commencement**"* or *"**Commencement Date**":* this Agreement will be deemed to have come into force on the Date of Commencement which shall be August 1, 2000, and shall be read and construed accordingly;

(f) *"**Effective Date of Termination**":* the date on which this Agreement is terminated pursuant to Article 18;

(g) *"**Field of Use**":* gene therapy being [**].

(h) *"**Xenon Improvements**":* improvements, variations, updates, modifications, and enhancements which relate to the Technology made solely by the Licensee or any sublicensees of the Licensee at any time after the Commencement Date that cannot be practised without infringing the claims of the Patents;

(i) *"**UBC Improvements**":* improvements, variations, updates, modifications, and enhancements which relate to the Technology made solely by the University at any time after the Commencement Date that cannot be practised without infringing the claims of the Patents;

(j) *"**Joint Improvements**":* improvements, variations, updates, modifications, and enhancements which relate to the Technology made jointly by the University and the Licensee or the University and any sublicensees of the Licensee at any time after the Commencement Date that cannot be practised without infringing the claims of the Patents;

(k) *"**Improvements**":* collectively the UBC Improvements, the Xenon Improvements and the Joint Improvements;

(l) *"**Information**":* any and all Technology and any and all Improvements, the terms and conditions of this Agreement and any and all oral, written, electronic or other communications and other information disclosed or provided by the parties including any and all analyses or conclusions drawn or derived therefrom regarding this Agreement and information developed or disclosed hereunder, or any party's raw materials, processes, formulations, analytical procedures, methodologies, products, samples and specimens or functions;

(m) *"**Patents**":* collectively the patents listed in Schedule *"**A**"*, including any patents or patent applications that may be added to Schedule *"**A**"* from time to time, and any counterparts, Continuation-ln-Part, renewals, divisionals, reissues, corresponding international patent applications, continuations and any patents resulting therefrom. For greater certainty the Patents and Patent applications as herein defined shall include, any and all Patents or Patent Applications arising from, or relating to Improvements, including Improvements that result from the Collaborative Research Agreement between the parties, which Patents or Patent applications shall be added to Schedule *"**A**";*

(n) *"**Product(s)**":* goods manufactured in connection with the use of all or some of the Technology and/or any Improvements;

(o) *"**Revenue**":*[**], less the following deductions to the extent included in the amounts invoiced and thereafter actually allowed and taken:

(i) [**],

(ii) [**],

(iii) [**],

(iv) [**], and

(v) [**].

Where any Revenue is derived from a country other than Canada it shall be converted to the equivalent in Canadian dollars on the date the Licensee is deemed to have received such Revenue pursuant to the terms hereof at the rate of exchange set by the Bank of Montreal for buying such currency. The amount of Canadian dollars pursuant to such conversion shall be included in the Revenue;

(p) *"**Royalty Due Dates**":* the last working day of March, June, September and December of each and every year during which this Agreement remains in full force and effect;

(q) *"**Sublicensing Revenue**":* [**], but excluding:

(i) [**] with Amsterdam Molecular Therapeutics B.V. *("**AMT**");*

(ii) [**] under the proposed sublicense with AMT [**];

(iii) research fees received by the Licensee in reimbursement for the actual costs of research and development undertaken by the Licensee pursuant to a written research plan.

Where any Sublicensing Revenue is derived from a country other than Canada it shall be converted to the equivalent in Canadian dollars on the date the Licensee is deemed to have received such Sublicensing Revenue pursuant to the terms hereof at the rate of, exchange set by the Bank of Montreal for buying such currency. The amount of Canadian dollars pursuant to such conversion shall be included in the Sublicensing Revenue;

(r) *"**Technology**":* any and all knowledge, know-how and/or technique or techniques invented, developed and/or acquired, prior to the Date of Commencement by the University relating to, and including the technology described in Schedule *"**A**"* hereto, as amended from time to time, including, without limitation the Patents, and collectively the University's interest in the UBC Technology, the UBC - [**] Technology and the UBC - Amsterdam Technology (as hereinafter defined in Article 2.1) and all research, data, specifications, instructions, manuals, papers or other materials of any nature whatsoever, whether written or otherwise, relating to same; and

(s) *"**UBC Trade-marks**":* any mark, trade-mark, service mark, logo, insignia, seal, design, symbol or device used by the University in any manner whatsoever.

2.0 PROPERTY RIGHTS IN AND TO THE TECHNOLOGY:

2.1. The parties hereto hereby acknowledge and agree that:

(a) Dr. Michael Hayden has assigned his rights to the Technology and any Improvements to the University;

(b) the University owns any and all right, title and interest in and to the technology identified in UBC Invention Disclosure File # UBC 94-061, entitled *"Lipolipase Mutation 291, Implication for Coronary Artery Disease"*, and File # UBC 91-003, entitled *"Mutation in Human Lipoprotein Lipase Gene which causes Type 1 Hyperlipoproteinemia"* as well as any and all UBC Improvements (the *"**UBC Technology**");*

(c) [**] has developed or acquired certain technology which has common subject matter with certain technology invented, developed and/or acquired by the University, and the University and [**] are named as joint owners within the United States of the technology identified in UBC Invention Disclosure File # UBC 99-082, entitled *"Recombinant Viruses Preparation and use thereof in Gene Therapy"* (the *"**UBC -[**] Technology**");*

(d) the University and AMC jointly own the technology identified in UBC Invention Disclosure File # UBC 00-039, entitled *"Mutation 447"* (the *"**UBC - Amsterdam Technology**"),*

(e) the University and the Licensee, subject to the terms of this Agreement jointly own all Joint Improvements, and provided that notwithstanding the applicable patent or other intellectual property laws of any jurisdiction both the University and the Licensee shall only use and commercially exploit any Joint Improvements in accordance with the terms of this Agreement; and

(f) the Licensee, subject to the terms of this Agreement, owns any all right, title and interest in and to the Xenon Improvements.

2.2. The Parties shall, on request, enter into such further agreements and execute any and all documents as may be required to ensure that ownership of the Technology, and any Improvements vest with, or remain with, the parties as set out in Article 2.1.

3.0 GRANT OF LICENSE:

3.1. In consideration of the license fees, milestone payments and royalty payments reserved herein, and the covenants on the part of the Licensee contained herein, the University hereby grants to the Licensee within the Field of Use:

(a) a worldwide exclusive license to use and sublicense the UBC Technology, any UBC Improvements or any Joint Improvements, and any Patents related thereto, including the right to manufacture, distribute, and sell Products and provide services on the terms and conditions hereinafter set forth during the term of this Agreement;

(b) a license of the University's rights to the UBC - [**] Technology and any UBC Improvements or Joint Improvements and any Patents related thereto, including the right to use and sublicense the University's rights in the UBC - [**] Technology and any UBC Improvements or Joint Improvements thereto, and to manufacture, distribute, and sell Products and provide services on the terms and

conditions hereinafter set forth during the term of this Agreement. The University acknowledges and agrees that it shall not license within the Field of Use any of the University's rights to the UBC - [**] Technology or any UBC Improvements or Joint Improvements thereto to any entity other than the Licensee for the duration of the term of this Agreement;

(c) a worldwide co-exclusive license together with Amsterdam Molecular Therapeutics B.V. *("**AMT**")* of the University's rights to the UBC - Amsterdam Technology and any UBC Improvements or Joint Improvements and any Patents related thereto, including the right to use and sublicense the University's rights to the UBC - Amsterdam Technology and any UBC Improvements or Joint Improvements thereto, and to manufacture, distribute, and sell Products and provide services on the terms and conditions hereinafter set forth during the term of this Agreement The Licensee acknowledges and agrees that AMC has granted a worldwide co-exclusive license to AMT to use and sublicense the UBC - Amsterdam Technology. The University acknowledges and agrees that it shall not license within the Field of Use any of the University's rights to the UBC - Amsterdam Technology or any UBC Improvements or Joint Improvements thereto to any entity other than the Licensee for the duration of the term of this Agreement;

3.2. The grant of the license:

(a) set out in Article 3.1(b) is made expressly subject to all of the rights which [**] has acquired to the UBC - [**] Technology. The Licensee hereby acknowledges that the use, practice, exploitation and commercialization of any rights to the UBC - [**] Technology may be subject to the consent of [**], and that it shall be the Licensee's sole responsibility to obtain such consent from [**];

(b) set out in Article 3.1(c) is expressly made subject to the conditions precedent that the written consent of AMC be obtained by the University prior to the grant of such a co-exclusive license to the Licensee.

In the event the University is unable to obtain the consent referred to in Article 3.2(b), the grant of license by the University to the Licensee herein shall be limited to the grant of license referred to in Article 3.1(a) and (b).

3.3. The licenses granted herein are personal to the Licensee and are not granted to any Affiliated Company or Affiliated Companies, subject to the right of the Licensee to sublicense as set out herein.

3.4. The Licensee shall not cross-license the Technology or any UBC Improvements or Joint Improvements without the prior written consent of the University.

3.5. Notwithstanding Article 3.1, and subject to Article 10.6 herein, the parties acknowledge and agree that the University may use the Technology and any Improvements (including UBC Improvements, Joint Improvements and Xenon Improvements) without charge in any manner whatsoever for research, scholarly publication, educational or other non-commercial uses. Except as expressly provided for above, the University may not use or license any Joint Improvements without the express, prior written consent of the Licensee.

4.0 SUBLICENSING:

4.1. The Licensee shall have the right to grant sublicenses to Affiliated Companies and other third parties with respect to the Technology and any Improvements with the prior written consent of the University, such consent not to be unreasonably withheld. The Licensee shall not be obligated to obtain the University's consent to the granting of a sublicense if the proposed sublicensee has a market capitalization in excess of CAN. $[**] at the time of the granting of the sublicense. Further, the University, subject to a full legal review and approval of the terms of such sublicense agreement and review of the performance terms in accordance with Article 11.3, hereby expressly consents to the Licensee granting a sublicense to AMT. The Licensee will furnish the University with a copy of each sublicense granted within 30 days after execution. The Licensee shall cause each sublicensee to indemnify the University on the same terms and conditions as are contained in Article 9.1 and which indemnity shall extend to cover any sub-sublicenses granted by such sublicensee.

4.2. Except as hereinafter provided, any sublicense granted by the Licensee shall be personal to the sublicensee and shall not be assignable without the prior written consent of the University, such consent not to be unreasonably withheld. A sublicensee may grant a further sub-sublicense to a third party for the purpose of developing, marketing, selling, manufacturing or distributing Products with the prior written consent of the University, such consent not to be unreasonably withheld. A sublicensee shall not be obligated to obtain the University's consent to the granting of a sub-sublicense if the proposed sub-sublicensee has a market capitalization in excess of CAN. $[**] at the time of the granting of the sub-sublicense. The sublicensee shall furnish the University with a copy of such sub-sublicense granted within 30 days after execution. A sublicense can be assigned without the consent of the University to an Affiliated Company of the sublicensee or as part of a merger, acquisition or other business combination in which all or substantially all of the assets of the sublicensee are transferred. All sublicenses and sub-sublicensees shall contain covenants by the sublicensee or sub-sublicensees to observe and perform the terms and conditions contained in this Agreement, to the extent that the same are applicable.

4.3. Upon execution of a sublicense with AMT, AMT may register such sublicense with the relevant patent authorities, in those jurisdictions in which AMT carries on business and/or has its chief place of business. The University will provide reasonable assistance to AMT with respect to such registrations,

provided that all reasonable costs incurred by the University in association with such registrations, including all legal expenses, shall be paid for by AMT, or the Licensee in the event of any default in payment by AMT. The University will, on request by the Licensee, endeavour to provide an estimate of such costs.

5.0 ROYALTIES:

5.1. In consideration of the license granted hereunder, the Licensee shall pay to the University a royalty comprised of:

(a) ·% of the Revenue, and

(b) ·% of the Sublicensing Revenue.

For clarification, Sublicensing Revenue shall be exclusive of Revenue, such that in no event shall the Licensee owe royalties to the University under both of Articles 5.1(a) and 5.1(b) in respect of any given amount of revenue.

5.2. If commercial development by the Licensee of a Product or Products incorporating the Technology or any Improvements is not possible without licensing other technology from an arms length third party to be used in combination with the Technology or any Improvements, then [**]:

- [**] and
- [**].

[**].

5.3. The royalty shall become due and payable within [**] days of each respective Royalty Due Date and shall be calculated with respect to the Revenue and the Sublicensing Revenue in the three month period immediately preceding the applicable Royalty Due Date.

5.4. All payments of royalties made by the Licensee to the University hereunder shall be made in Canadian dollars without any reduction or deduction of any nature or kind whatsoever, except as may be prescribed by Canadian law.

5.5. Products shall be deemed to have been sold by the Licensee and included in the Revenue when invoiced, or if not invoiced, then when delivered, shipped, or paid for, whichever is the first. Sublicensing Revenue shall be deemed to have been received by the Licensee with respect to each of its sublicensees when such consideration is actually received by the Licensee from its sublicensees.

5.6. Any transaction, disposition, or other dealing involving the Technology or any part thereof between the Licensee and another person that is not made at fair market value shall be deemed to have been made at fair market value, and the fair market value of that transaction, disposition, or other dealing shall be added to and deemed part of the Revenue or the Sublicensing Revenue, as the case may be, and shall be included in the calculation of royalties under this Agreement.

6.0 INITIAL LICENSE FEE, ANNUAL MAINTENANCE FEE AND MINIMUM ANNUAL ROYALTY:

6.1. As part of the consideration for the rights granted by the University to the Licensee hereunder, the Licensee agrees to issue to the University, as an initial license fee the sum of $· (Canadian funds) (the *"**Initial License Fee**")*. The said sum shall be paid concurrently with the execution of this Agreement. Neither all nor any portion of the said sum shall be refundable to the Licensee under any circumstances.

6.2. The Licensee acknowledges and agrees that the University has agreed to accept the Initial License Fee on the condition that[**].

6.3. In further consideration for the license granted hereunder, the Licensee shall pay to the University, in addition to all other amounts due under this Agreement, an annual maintenance fee of CAN. $· payable on execution of this Agreement and thereafter on or before September 1st of each year during which this Agreement remains in full force and effect (the *"**Annual Maintenance Fee**")*. Neither all nor any part of the Annual Maintenance Fee paid shall be refundable to the Licensee under any circumstances.

6.4. In addition to all other payments due hereunder, the Licensee shall pay to the University the following milestones, for each product developed by the Licensee or a sublicensee:

(a) within [**] days of [**] the sum of CAN. $·;

(b) within [**] days of[**], the sum of CAN. $·; and

(c) within [**] days of receipt of [**], the sum of CAN. $·.

For greater clarity, it is agreed that the foregoing milestone payments shall be due and payable by the Licensee regardless of whether such milestones are achieved by the Licensee or a sublicensee, and such milestone payments shall in no way effect or diminish the royalties which are due and payable hereunder, and in particular, the calculation of the amount of royalty payable in connection with the Sublicensing Revenue.

6.5. The Licensee shall pay to the University the milestones due under Article 6.4. in the timeframes provided under Article 6.4, with the following exceptions:

(a) [**];

(b) [**]:

(i) [**], or

(ii) [**]; and

6.6. For greater certainty, and notwithstanding any provisions within this Agreement to the contrary, the parties agree that:

(a) [**]; and

(b) [**].

7.0 PATENTS:

7.1. The Licensee shall have the right to identify any process, use or products arising out of the Technology and any UBC Improvements or any Joint Improvements that may be patentable including the right to apply for further patents in other jurisdictions, or continuations, continuations-in-part, divisions, reissues, re-examinations or extensions of the Patents or any further applications made hereunder, and shall take all reasonable steps to apply for such a patents in the name of the University or jointly in the names of the University and the Licensee in the case of any patent relating to a Joint Improvement, provided that the Licensee pays all costs of applying for, registering and maintaining the patent in such jurisdictions as the Licensee may designate. The Licensee shall be responsible for the management, filing, prosecution and maintenance of such Patents, provided however, that the Licensee will obtain the University's prior consent, as to any material decision or action taken in the prosecution of such Patents, which consent shall not to be unreasonably withheld by the University. The Licensee shall also provide the University with copies of all correspondence and documents relating to the filing, prosecution and maintenance of the Patents. In the event that this Agreement is terminated for any reason whatsoever, the Licensee shall pay all outstanding costs relating to such patent

applications to the date of termination and shall direct the patent agents responsible for such patent applications to take all further instructions, if any, relating to such applications from the University.

7.2. On the issuance of a patent in accordance with Article 7.1, the Licensee shall have the right to become, and shall become, the licensee of the same all pursuant to the terms contained herein.

7.3. As of April 27, 2001, the University has incurred CAN. $80,091.70 in patenting the Technology. On execution of this Agreement, the Licensee will pay to the University the sum of CAN. $80,091.70 to reimburse the University for these costs. All further costs with respect to all Patents or Patent Applications relating to the Technology and any UBC Improvements or Joint Improvements, and all maintenance fees for such patents incurred by the University at any time after April 27, 2001, shall be reimbursed by the Licensee to the University within 30 days of presentation of receipts and/or invoices by the University to the Licensee. Without limiting the generality of the forgoing the Licensee agrees to pay for all costs with respect to the Patents, patent applications, divisionals, substitutions, continuations, continuations in part, all claims of foreign patent applications.

7.4. Should the Licensee decide to:

(a) discontinue pursuing patent protection in relation to the Patents, or any continuation, continuation-in-part, division, re-issue, re-examination or extension of the Patent(s), or

(b) not pursue patent protection in relation to the Patent(s) in any jurisdiction, or

(c) discontinue or not pursue patent protection in relation to any further process, use or products arising out of the UBC Improvements or Joint Improvements in any jurisdiction,

then the Licensee shall provide the University with a minimum of [**] days notice of its decision to discontinue or not to pursue such patent protection in sufficient time for the University to file a patent application, or continue pursuing an existing patent application. During the [**] day transition period the Licensee shall be responsible for all costs of filing, prosecuting and maintaining the Patents.

7.5. The Licensee shall provide to the University [**].

7.6. [**].

7.7. The Licensee will ensure proper patent marking for all Technology, and any UBC Improvements or Joint Improvements licensed hereunder and shall clearly mark the appropriate patent numbers on any Products made using the Technology and any UBC Improvements or Joint Improvements or any patented processes used to make such Products.

8.0 DISCLAIMER OF WARRANTY:

8.1. The University makes no representations, conditions or warranties, either express or implied, with respect to the Technology or any Improvements or the Products.

Without limiting the generality of the foregoing, the University specifically disclaims any implied warranty, condition or representation that the Technology or any Improvements or the Products:

(a) shall correspond with a particular description;

(b) are of merchantable quality;

(c) are fit for a particular purpose; or

(d) are durable for a reasonable period of time.

The University shall not be liable for any loss, whether direct, consequential, incidental or special, which the Licensee suffers arising from any defect, error, fault or failure to perform with respect to the Technology or any Improvements or Products, even if the University has been advised of the possibility of such defect, error, fault or failure. The Licensee acknowledges that it has been advised by the University to undertake its own due diligence with respect to the Technology and any Improvements.

8.2. The parties acknowledge and agree that the *International Sale of Goods Contracts Convention Act* and the United Nations Convention on Contracts for the International Sale of Goods have no application to this Agreement.

8.3. Nothing in this Agreement shall be construed as:

(a) a warranty or representation by the University as to title to the Technology and/or any Improvement or that anything made, used, sold or otherwise disposed of under the license granted in this Agreement is or will be free from infringement of patents, copyrights, trade-marks, industrial design or other intellectual property rights;

(b) an obligation by the University to bring or prosecute or defend actions or suits against third parties for infringement of patents, copyrights, trade-marks, industrial designs or other intellectual property or contractual rights; or

(c) the conferring by the University of the right to use in advertising or publicity the name of the University or the UBC Trade-marks.

8.4. Notwithstanding Article 8.3, in the event of an alleged infringement of the Technology or any UBC Improvements or Joint Improvements or any right with respect to the Technology or any UBC Improvements or Joint Improvements, the Licensee or a sublicensee shall have, upon receiving the prior written consent of the University, such consent not to be unreasonably withheld, the right but not the obligation to prosecute litigation designed to enjoin infringers of the Technology or any UBC Improvements or Joint Improvements. The Licensee acknowledges and agrees that the University may require the consent of [**] and/or AMC, as appropriate (in so far as any UBC - [**] Technology or UBC - Amsterdam Technology is alleged to be infringed), prior to providing such consent. Provided that it has first granted its prior written consent, the University agrees to co-operate to the extent of executing all necessary documents and to vest in the Licensee or sublicensee the right to institute any such suits, so long as all the direct and indirect costs and expenses of bringing and conducting any such litigation or settlement shall be borne by the Licensee or sublicensee and in such event all recoveries shall enure to the Licensee or sublicensee.

8.5. If any complaint alleging infringement or violation of any patent or other proprietary rights is made against the Licensee or a sublicensee of the Licensee with respect to the use of the Technology or any UBC Improvements or Joint Improvements or the manufacture, use or sale of the Products, the following procedure shall be adopted:

(a) the Licensee shall promptly notify the University upon receipt of any such complaint and shall keep the University fully informed of the actions and positions taken by the complainant and taken or proposed to be taken by the Licensee on behalf of itself or a sublicensee;

(b) except as provided in Article 8.5(d), all costs and expenses incurred by the Licensee or any sublicensee of the Licensee in investigating, resisting, litigating and settling such a complaint, including the payment of any award of damages and/or costs to any third party, shall be paid by the Licensee or any sublicensee of the Licensee, as the case may be;

(c) no decision or action concerning or governing any final disposition of the complaint shall be taken without full consultation with and approval by the University;

(d) the University may elect to participate formally in any litigation involving the complaint to the extent that the court may permit, but any additional expenses generated by such formal participation shall be paid by the University (subject to the possibility of recovery of some or all of such additional expenses from the complainant);

(e) notwithstanding Article 8.3, if the complainant is willing to accept an offer of settlement and one of the parties to this Agreement is willing to make or accept such offer and the other is not, then the unwilling party shall conduct all further proceedings at its own expense, and shall be responsible for the full amount of any damages, costs, accounting of profits and settlement costs in excess of those provided in such offer, but shall be entitled to retain unto itself the benefit of any litigated or settled result entailing a lower payment of costs, damages, accounting of profits and settlement costs than that provided in such offer; and

(f) the royalties payable pursuant to this Agreement shall be paid by the Licensee to the University in trust from the date the complaint is made until such time as a resolution of the complaint has been finalized. If the complainant prevails in the complaint, then the royalties paid to the University in trust pursuant to this Article shall be returned to the Licensee, provided that the amount returned to the Licensee hereunder shall not exceed the amount paid by the Licensee to the complainant in the settlement or other disposition of the complaint. If the complainant does not prevail in the complaint, then the University shall be entitled to retain all royalties paid to it pursuant to this Article.

9.0 INDEMNITY AND LIMITATION OF LIABILITY:

9.1. The Licensee hereby indemnifies, holds harmless and defends the University, its Board of Governors, officers, employees, faculty, students, invitees and agents against any and all claims (including all legal fees and disbursements incurred in association therewith) collectively a ***"Claim")*** arising out of the exercise of any rights under this Agreement including,

without limiting the generality of the foregoing, against any damages or losses, consequential or otherwise, arising from or out of the use of the Technology or Products licensed under this Agreement by the Licensee or its sublicensees, and sub-sublicensees, or their customers or end-users howsoever the same may arise. A condition of this obligation is that, whenever the University has information from which it may reasonably conclude an incident has occurred which could give

rise to a Claim, the University shall promptly give notice to the Licensee of all pertinent data surrounding such incident and, in the event a Claim is made or suit is brought the University shall assist the Licensee and cooperate in the gathering of information with respect to the time, place and circumstances and in obtaining the names and addresses of any injured parties and available witnesses. The University shall not voluntarily make any payment or incur any expense in connection with any such Claim without the prior written consent of the Licensee. The Licensee shall have control over the defence and settlement of any Claim, provided that the Licensee keeps the University informed of all activities in a timely manner. The obligations set forth in this Article 9.1 shall survive the expiration or termination of this Agreement.

9.2. Subject to Article 9.3, the University's total liability, whether under the express or implied terms of this Agreement, in tort (including negligence), or at common law, for any loss or damage suffered by the Licensee, whether direct, indirect or special, or any other similar or like damage that may arise or does arise from any breaches of this Agreement by the University, its Board of Governors, officers, employees, faculty, students or agents, shall be limited to the amount of the Initial License Fee paid pursuant to Article 6.1.

9.3. In no event shall the University be liable for consequential or incidental damages arising from any breach or breaches of this Agreement.

9.4. No action, whether in contract or tort (including negligence), or otherwise arising out of or in connection with this Agreement, may be brought by the Licensee more than six months after the cause of action has occurred.

10.0 PUBLICATION AND CONFIDENTIALITY:

10.1. The Information provided by the University shall be developed, received and used by the Licensee solely in furtherance of the purposes set forth in this Agreement subject to the terms and conditions set forth in this Article 10.

10.2. Each party hereto covenants and agrees that it will initiate and maintain an appropriate internal program limiting the internal distribution of the other party's Confidential Information to only those officers, employees and professional advisors who require said Confidential Information in performing their obligations under this Agreement and who have signed confidentiality and non-disclosure agreements in a form approved by the Licensee's Board of Directors in the case of the Licensee and in a form consistent with the terms of this Agreement in the case of the University.

10.3. Subject to Article 10.8, the Licensee and the University shall not use, either directly or indirectly, any Confidential Information of the other party for any purpose other than as set forth herein without the other party's prior written consent.

10.4. If the Licensee or the University are required by judicial or administrative process to disclose any or all of the other party's Confidential Information, they shall promptly notify the

other party and allow the other party reasonable time to oppose such process before disclosing any such Confidential Information.

10.5. Notwithstanding any termination or expiration of this Agreement, the obligations created in this Article 10 shall survive and be binding upon the Licensee and the University, and their successors and assigns.

10.6. The University shall not be restricted from presenting at symposia, national or regional professional meetings, or from publishing in journals or other publications, accounts of its research relating to the Information, provided that with respect to Confidential Information only, the Licensee shall have been furnished copies of the disclosure proposed therefor at least [**] days in advance of the presentation or publication date and does not within [**] days after receipt of the proposed disclosure object to such presentation or publication. Any objection to a proposed presentation or publication shall specify the portions of the presentation or publication considered objectionable (the *"**Objectionable Material**").* Upon receipt of notification from the Licensee that any proposed publication or disclosure contains Objectionable Material, the University and the Licensee shall work together to revise the proposed publication or presentation to remove or alter the Objectionable Material in a manner acceptable to the Licensee, in which case the Licensee shall withdraw its objection. If an objection is made, disclosure of the Objectionable Material shall not be made for a period of [**] months after the date the Licensee has received the proposed publication or presentation relating to the Objectionable Material. The University shall co-operate in all reasonable respects in making revisions to any proposed disclosures if considered by the Licensee to contain Objectionable Material. The University shall not be restricted from publishing or presenting the proposed disclosure as long as the Objectionable Material has been removed. After the 6 month period has elapsed the University shall be free to present and/or publish the proposed publication or presentation whether or not it contains Objectionable Material.

10.7. Subject to Article 10.8, the Licensee requires of the University, and the University agrees insofar as it may be permitted to do so at law, that this Agreement, and each part of it, is confidential and shall not be disclosed to third parties, as the Licensee claims that such disclosure would or could reveal commercial, scientific or technical information and would significantly harm the Licensee's competitive position and/or interfere with the Licensee's negotiations with prospective sublicensees. Notwithstanding anything contained in this Article, the parties hereto acknowledge and agree that the University may identify the title of this Agreement, the parties to this Agreement and the names of the inventors of the Technology and any Improvements.

10.8. Notwithstanding the forgoing, the parties acknowledge and agree that the University and the Licensee may provide a copy of this Agreement to AMC, AMT and [**], and the University must provide certain reports and information to its Board of Governors, the Province of British Columbia and the government of Canada which may inter alia, include a summary of the terms of this Agreement and the activities thereunder.

11.0 PRODUCTION AND MARKETING:

11.1. The Licensee shall not use any of the UBC Trade-marks or make reference to the University or its name in any advertising or publicity whatsoever, without the prior written consent of the University, except as required by law and except that the Licensee and any of its sublicensees may disclose the existence and nature of this Agreement and (subject to the confidentiality provisions of Article 10) the nature of the technology being licensed without the

need for the University's consent. Without limiting the generality of the foregoing, the Licensee shall not issue a press release with respect to this Agreement or any activity contemplated herein without the prior review and approval of same by the University, which approval shall not be unreasonably withheld, except as

required by law. If the Licensee is required by law to act in contravention of this Article, to the extent permissible by law, the Licensee shall provide the University with sufficient advance notice in writing to permit the University to bring an application or other proceeding to contest the requirement.

11.2. The Licensee will not register or use any UBC Trade-marks in association with the Products without the prior written consent of the University.

11.3. The Licensee shall use its commercially reasonable efforts to[**]. The University acknowledges and agrees that subject to the University's prior review and approval of the terms in the contemplated sublicense between the Licensee and AMT pursuant to Article 4.1, the granting of such a sublicense by the Licensee to AMT will meet the forgoing obligation of the Licensee. Without limiting the generality of the foregoing, the Licensee covenants and agrees that it shall provide to the University, on each of the first five anniversaries of the Commencement Date of the License Agreement or the date of an amendment to the License Agreement, a written report (the *"**Status Report**")* summarizing the Licensee's development activities relating to the Technology and any Improvements that sets out all of the following information:

(a) a summary of the research and development activities that the Licensee has undertaken in the course of the preceding 12 months to develop and commercialize the Technology and any Improvements;

(b) a detailed summary of any and all improvements, variations, updates, modifications and enhancements to the Technology and any Improvements which the Licensee has developed and/or acquired in the course of the preceding 12 months, including any improvements, variations, updates, modifications and enhancements to the Technology or any Improvements of which the Licensee has been advised by any sublicensee, or sub-sublicensee of the Licensee; and

(c) any and all corporate alliances formed by the Licensee related to the Technology or any Improvements in the course of the preceding 12 months, including any such corporate alliances of which the Licensee has been advised by a sublicensee or sub-sublicensee of the Licensee.

11.4. If the University is of the view that the Licensee is in material breach of Article 11.3, then the University shall notify the Licensee and the parties hereto shall appoint a mutually acceptable person as an independent evaluator (the *"**Evaluator**") **to*** conduct the evaluation set forth in Article 11.3. If that the parties cannot agree on such an Evaluator, the appointing authority shall be the British Columbia International Commercial Arbitration Centre.

11.5. Unless the Parties mutually agree otherwise, the following rules and procedures shall govern the conduct of the parties and the Evaluator before and during the investigation by the Evaluator:

(a) within [**] days of the appointment of the Evaluator each party shall provide to the Evaluator and the other party copies of all documents, statements and

records on which the party intends to rely in presenting its position to the Evaluator;

(b) within [**] days of the appointment of the Evaluator the Licensee shall provide to the Evaluator and the University a written summary of its position. On receipt of the Licensee's summary the University shall have [**] days to prepare and submit to the Licensee and the Evaluator its own summary in reply to the summary submitted by the Licensee;

(c) on receipt of the documents, statements, records and summaries submitted by the parties the Evaluator shall have [**] days within which to conduct such further inquiries as he or she may deem necessary for the purpose of reviewing the efforts made by the Licensee with respect to the promotion, marketing and sale of the Products and the Technology and any Improvements in compliance with the requirements of Article 11.3. For the purpose of conducting such an inquiry, the Evaluator shall have the right to:

(i) require either party to disclose any further documents or records which the Evaluator considers to be relevant;

(ii) interview or question either orally (or by way of written questions) one or more representatives of either party on issues deemed to be relevant by the Evaluator;

(iii) make an *"on site"* inspection of the Licensee's facilities;

(iv) obtain if necessary, the assistance of an independent expert to provide technical information with respect to any area in which the Evaluator does not have a specific expertise;

(d) On completion of the Inquiry described in Article 11.5(c) the Evaluator shall within [**] days prepare a report setting out his or her findings and conclusions as to whether or not the Licensee has committed a breach of Article 11.3. If the Evaluator has determined that the Licensee has committed a breach of Article 11.3, then the Evaluator shall also set out in the report his or her conclusions as to whether such breach:

(i) was substantially due to external market conditions not within the control of the Licensee, or

(ii) was substantially due to the Licensee's failure to use its commercially reasonable efforts to comply with the requirements of Article 11.3.

(e) The report and conclusions of the Evaluator shall be delivered to the Licensee and the University, and shall be accepted by both parties as final and binding.

11.6. If the Evaluator concludes:

(a) pursuant to Article 11.5(d)(i) that the Licensee's material breach was substantially due to external market conditions and not due to any omission or failure on the

part of the Licensee, then the License granted hereunder shall continue in good standing,

(b) pursuant to Article 11.5(d)(ii) that the Licensee's material breach was substantially due to the Licensee's failure to use commercially reasonable efforts then the University shall at its option have the right to terminate this Agreement as provided in Article 18, or

(c) pursuant to Article 11.5(d) that the Licensee is not in material breach of Article 11.3, then the University shall not terminate this Agreement for breach of Article 11.3, nor shall it change the nature of the license granted hereunder.

11.7. The University may not call for more than one evaluation pursuant to Article 11.4 in each calendar year. The cost of an evaluation hereunder shall be borne [**].

12.0 ACCOUNTING RECORDS:

12.1. The Licensee shall maintain at its principal place of business, or such other place as may be most convenient, separate accounts and records of all Revenues, sublicenses and Sublicensing Revenues, and all business done pursuant to this Agreement, such accounts and records to be in sufficient detail to enable proper returns to be made under this Agreement, and the Licensee shall cause its sublicensees to keep similar accounts and records.

12.2. The Licensee shall deliver to the University on the date [**] days after each and every Royalty Due Date, together with the royalty payable thereunder, the Accounting and a report on all Sublicensing activity, including an accounting statement setting out in detail how the amount of Sublicensing Revenue was determined and identifying each sublicensee and the location of the business of each sublicensee.

12.3. The calculation of royalties shall be carried out in accordance with generally accepted Canadian accounting principles *("**GAP**")*, or the standards and principles adopted by the U.S. Financial Accounting Standards Board *("**FASB**")* applied on a consistent basis.

12.4. The Licensee shall retain the accounts and records referred to in Article 12.1 above for at least [**] years after the date upon which they were made and shall permit any duly authorized representative of the University to inspect such accounts and records during normal business hours of the Licensee at the University's expense. The Licensee shall furnish such reasonable evidence as such representative will deem necessary to verify the Accounting and will permit such representative to make copies of or extracts from such accounts, records and agreements at the University's expense. If an inspection of the Licensee's records by the University shows an under-reporting or underpayment by the Licensee of any amount to the University, in excess of [**]% for any [**] month period, then the Licensee shall reimburse the University for the cost of the inspection as well as pay to the University any amount found due (including any late payment charges or interest) within [**] days of notice by the University to the Licensee.

12.5. During the term of this Agreement, and thereafter, [**].

13.0 INSURANCE:

13.1. Unless satisfactory arrangements are made between the Licensee and the University with respect to a self-insurance program or the requirement for insurance hereunder is waived by the University [**] days prior to the commencement of any human clinical trials or other Product testing involving human subjects by the Licensee or any sublicensee, then the Licensee shall procure and maintain, during the term of this Agreement, the insurance outlined in Articles 13.2 and 13.3 and otherwise comply with the insurance provisions contained in Articles 13.2 and 13.3.

13.2. The Licensee shall give written notice to the University:

(a) [**] days prior to the commencement of any human clinical trials or other Product testing involving human subjects by the Licensee or any sublicensee, *("**Human Clinical Trials**");* and

(b) [**] days prior to the first sale of any Product by the Licensee or any sublicensee, of the terms and amount of the appropriate public liability, product liability and errors and omissions insurance which it has placed. Such insurance shall in no case be less than the insurance which a reasonable and prudent businessperson carrying on a similar line of business would acquire. This insurance shall be placed with a reputable and financially secure insurance carrier, shall include the University, its Board of Governors, faculty, officers, employees, students, and agents as additional insureds, and shall provide primary coverage with respect to the activities contemplated by this Agreement. Such policy shall include severability of interest and cross-liability clauses and shall provide that the policy shall not be cancelled or materially altered except upon at least [**] days' written notice to the University. The University shall have the right to require reasonable amendments to the terms or the amount of coverage contained in the policy. Failing the parties agreeing on the appropriate terms or the amount of coverage, then the matter shall be determined by arbitration. The Licensee shall provide the University with certificates of insurance evidencing such coverage [**] days before commencement of Human Clinical Trials and [**] days prior to the sales of any Product and the Licensee covenants not to start Human Clinical Trials, or sell any Product before such certificate is provided and approved by the University, or to sell any Product at any time unless the insurance outlined in this Article 13.2 is in effect.

13.3. The Licensee shall require that each sublicensee under this Agreement shall procure and maintain, during the term of the sublicense, public liability, product liability and errors and omissions insurance in reasonable amounts, with a reputable and financially secure insurance carrier or provide satisfactory arrangements through an appropriate self-insurance program. The Licensee shall use its best efforts to ensure that any and all such policies of insurance required pursuant to this Article shall contain a waiver of subrogation against the University, its Board of Governors, faculty, officers, employees, students, and agents.

14.0 ASSIGNMENT:

14.1. Except as hereinafter provided, the Licensee will not assign, transfer, mortgage, charge or otherwise dispose of any or all of the rights, duties or obligations granted to it under this Agreement without the prior written consent of the University, (subject to the Licensee's

right to sublicense without the prior written consent of the University pursuant to Article 4.1), such consent not to be unreasonably withheld. The Licensee may assign this license without the consent of the University as part of a merger, acquisition or other business combination in which all or substantially all of the assets of the Licensee are transferred.

14.2. The University shall have the right to assign its rights, duties and obligations under this Agreement to a company or society of which it is the sole shareholder, in the case of a company, or of which it controls the membership, in the case of a society. In the event of such an assignment, the Licensee will release, remise and forever discharge the University from any and all obligations or covenants, provided however that such company or society, as the case may be, executes a written agreement which provides that such company or society shall assume all such obligations or covenants from the University and that the Licensee shall retain all rights granted to the Licensee pursuant to this Agreement.

15.0 GOVERNING LAW:

15.1. This Agreement shall be governed by and construed in accordance with the laws of the Province of British Columbia and the laws of Canada in force therein without regard to its conflict of law rules.

16.0 NOTICES:

16.1. All payments, reports and notices or other documents that any of the parties hereto are required or may desire to deliver to any other party hereto may be delivered only by personal delivery or by registered or certified mail, telex or fax, all postage and other charges prepaid, at the address for such party set forth below or at such other address as any party may hereinafter designate in writing to the others. Any notice personally delivered or sent by telex or fax shall be deemed to have been given or received at the time of delivery, telexing or faxing. Any notice mailed as aforesaid shall be deemed to have been received on the expiration of five days after it is posted, provided that if there shall be at the time of mailing or between the time of mailing and the actual receipt of the notice a mail strike, slow down or labour dispute which might affect the delivery of the notice by the mails, then the notice shall only be effected if actually received.

If to the University:
The Managing Director
University - Industry Liaison Office
University of British Columbia
IRC 331 - 2194 Health Sciences Mall
Vancouver, British Columbia
V6T 1Z3
Telephone: (604)822-8580
Fax: (604)822-8589

If to the Licensee:
The President
Xenon Genetics Inc.
Gerald McGavin Building
Suite 100 - 2386 East Mall
Vancouver, British Columbia
V6T 1Z3
Telephone: (604)221-8478
Fax: (604)221-8423

17.0 TERM:

17.1. This Agreement and the license granted hereunder shall terminate on the expiration of a term of 10 years from the Date of Commencement or the expiration of the last patent obtained pursuant to Article 7 herein, whichever event shall last occur, unless earlier terminated pursuant to Article 18 herein.

18.0 TERMINATION:

18.1. This Agreement shall automatically and immediately terminate without notice to the Licensee if any proceeding under the *Bankruptcy and Insolvency Act* of Canada, or any other statute of similar purport, is commenced by or against the Licensee provided such proceedings have not been dismissed within [**] days of the date on which they were commenced. In the event that the sublicense to be entered into between the Licensee and AMT is terminated, the Licensee may terminate this Agreement on [**] days prior written notice to the University, subject to payment of all amounts owed to the University.

18.2. The University may, at its option, terminate this Agreement immediately on the happening of any one or more of the following events by delivering notice in writing to that effect to the Licensee:

(a) if any resolution is passed or order made or other steps taken for the winding up, liquidation or other termination of the existence of the Licensee;

(b) if the Licensee is more than [**] days in arrears of royalties or other monies that are due to the University under the terms of this Agreement after written notice;

(c) if the Technology or any Improvements becomes subject to any security interest, charge or encumbrance in favour of any third party , other than a sublicensee, granted by the Licensee without prior written consent of the University, not to be unreasonably withheld;

(d) if the Licensee ceases or threatens to cease to carry on its business;

(e) if the Licensee undergoes a reorganization or any part of its business relating to this Agreement is transferred to a subsidiary or associated company without the prior written consent of the University, such consent not to be withheld except as provided in Article 18.3 and to be provided within [**] days of receipt of a written request for the same;

(f) if the Licensee commits any breach of Articles 4.1, 11.1, 11.2 or 13;

(g) if it is determined, pursuant to Article 11.5, that the Licensee is in breach of Article 11.3;

(h) if any sublicensee of the Licensee is in breach of its sublicense agreement with the Licensee and the Licensee does not cause such sublicensee to cure such default within [**] days of receipt of written notice from the University requiring that the Licensee cause such sublicensee to cure such default, or

(i) if the Licensee is in breach of the Collaborative Research Agreement dated August 1, 2000, between the Licensee and the University, which breach has not been cured within the time provided for the curing of such breach under the terms of such other agreement.

18.3. The University shall not withhold its consent pursuant to Article 18.2(e) unless the granting of such consent would result in the University having a contractual relationship with an entity with whom the University is prohibited from contracting with pursuant to its then existing policies.

18.4. Other than as set out in Articles 18.1 and 18.2, if either party shall be in default under or shall fail to comply with the terms of this Agreement then the non-defaulting party shall have the right to terminate this Agreement by written notice to the other party to that effect if:

(a) such default is reasonably curable within [**] days after receipt of notice of such default and such default or failure to comply is not cured within 30 days after receipt of written notice thereof; or

(b) such default is not reasonably curable within [**] days after receipt of written notice thereof, and such default or failure to comply is not cured within such further reasonable period of time as may be necessary for the curing of such default or failure to comply.

Any written notice issued pursuant to this Article 18.5 shall expressly set out the default or defaults with respect to which notice is being given.

18.5. If this Agreement is terminated pursuant to Article 18.1, 18.2, or 18.4, the Licensee shall make royalty payments to the University in the manner specified in Article 5, and 6 and the University may proceed to enforce payment of all outstanding royalties or other monies owed to the University and to exercise any or all of the rights and remedies contained herein or otherwise available to the University by law or in equity, successively or concurrently, at the option of the University. Upon any such termination of this Agreement, the Licensee shall forthwith deliver up to the University all Technology and any UBC Improvements in its possession or control and shall have no further right of any nature whatsoever in the Technology or any UBC Improvements. On the failure of the Licensee to so deliver up the Technology and any UBC Improvements, the University may immediately and without notice enter the Licensee's premises and take possession of the Technology and any UBC Improvements. The Licensee will pay all charges or expenses incurred by the University in the enforcement of its rights or remedies against the Licensee including, without limitation, the University's legal fees and disbursements on an indemnity basis.

18.6. The Licensee shall cease to use the Technology or any UBC Improvements in any manner whatsoever or to manufacture or sell the Products within five days from the Effective Date of Termination, subject to the expiration or invalidation of any applicable Patents. The Licensee shall then deliver or cause to be delivered to the University an accounting within 30 days from the Effective Date of Termination. The accounting will specify, in or on such terms as the University may in its sole discretion require, the inventory or stock of Products manufactured and remaining unsold on the Effective Date of Termination. The University will instruct that the unsold Products be stored, destroyed or sold under its direction, provided this Agreement was terminated by the University pursuant to Article 18.2 or 18.4 and subject to the expiration or invalidation of any applicable Patents. Without limiting the generality of the

foregoing, if this Agreement was terminated pursuant to Article 18.1, the unsold Products will not be sold by any party without the prior written consent of the University. The Licensee will continue to make royalty payments to the University in the same manner specified in Article 5 and 6 on all unsold Products that are sold in accordance with this Article 18.6, notwithstanding anything contained in or any exercise of rights by the University under Article 18.5 herein.

18.7. Notwithstanding the termination of this Agreement, Article 12 shall remain in full force and effect until [**] years after

(a) all payments of royalty required to be made by the Licensee to the University under this Agreement have been made by the Licensee to the University, and

(b) any other claim or claims of any nature or kind whatsoever of the University against the Licensee has been settled.

19.0 MISCELLANEOUS COVENANTS OF LICENSEE:

19.1. The Licensee hereby represents and warrants to the University that the Licensee is a corporation duly organized, existing and in good standing under the laws of Canada and has the power, authority and capacity to enter into this Agreement and to carry out the transactions contemplated by this Agreement, all of which have been duly and validly authorized by all requisite corporate proceedings.

19.2. The Licensee represents and warrants that [**].

19.3. The Licensee shall comply with all laws, regulations and ordinances, whether Federal, State, Provincial, County, Municipal or otherwise, with respect to the Technology and any Improvements and/or this Agreement.

19.4. [**].

19.5. [**].

19.6. The Licensee shall pay all taxes and any related interest or penalty howsoever designated and imposed as a result of the existence or operation of this Agreement, including, but not limited to, tax which the Licensee is required to withhold or deduct from payments to the University. The Licensee will furnish to the University such evidence as may be required by Canadian authorities to establish that any such tax has been paid. The royalties specified in this Agreement are exclusive of taxes. If the University is required to collect a tax to be paid by the Licensee or any of its sublicensees, the Licensee shall pay such tax to the University on demand.

19.7. The obligation of the Licensee to make all payments hereunder will be absolute and unconditional and will not, except as expressly set out in this Agreement, be affected by any circumstance, including without limitation any set-off, compensation, counterclaim, recoupment, defence or other right which the Licensee may have against the University, or anyone else for any reason whatsoever.

19.8. All amounts due and owing to the University hereunder but not paid by the Licensee on the due date thereof shall bear interest in Canadian dollars at the rate of one per cent (1%) per month. Such interest shall accrue on the balance of unpaid amounts from time to

time outstanding from the date on which portions of such amounts become due and owing until payment thereof in full.

20.0 GENERAL:

20.1. Nothing contained herein shall be deemed or construed to create between the parties hereto a partnership or joint venture. No party shall have the authority to act on behalf of any other party, or to commit any other party in any manner or cause whatsoever or to use any other party's name in any way not specifically authorized by this Agreement. No party shall be liable for any act, omission, representation, obligation or debt of any other party, even if informed of such act, omission, representation, obligation or debt.

20.2. Subject to the limitations hereinbefore expressed, this Agreement shall enure to the benefit of and be binding upon the parties and their respective successors and permitted assigns.

20.3. No condoning, excusing or overlooking by any party of any default, breach or non- observance by any other party at any time or times in respect of any covenants, provisos or conditions of this Agreement shall operate as a waiver of such party's rights under this Agreement in respect of any continuing or subsequent default, breach or non-observance, so as to defeat in any way the rights of such party in respect of any such continuing or subsequent default or breach, and no waiver shall be inferred from or implied by anything done or omitted by such party, save only an express waiver in writing.

20.4. No exercise of a specific right or remedy by any party precludes it from or prejudices it in exercising another right or pursuing another remedy or maintaining an action to which it may otherwise be entitled either at law or in equity.

20.5. Marginal headings as used in this Agreement are for the convenience of reference only and do not form a part of this Agreement and are not be used in the interpretation hereof.

20.6. The terms and provisions, covenants and conditions contained in this Agreement which by the terms hereof require their performance by the parties hereto after the expiration or termination of this Agreement shall be and remain in force notwithstanding such expiration or other termination of this Agreement for any reason whatsoever.

20.7. If any Article, part, section, clause, paragraph or subparagraph of this Agreement shall be held to be indefinite, invalid, illegal or otherwise voidable or unenforceable, the entire Agreement shall not fail on account thereof, and the balance of this Agreement shall continue in full force and effect.

20.8. The parties hereto each acknowledge that the law firm of Richards Buell Sutton has acted solely for the University in connection with this Agreement and that all other parties hereto have been advised to seek independent legal advice.

20.9. This Agreement sets forth the entire understanding between the parties and no modifications hereof shall be binding unless executed in writing by the parties hereto.

20.10. Time shall be of the essence of this Agreement.

20.11. Whenever the singular or masculine or neuter is used throughout this Agreement the same shall be construed as meaning the plural or feminine or body corporate when the context or the parties hereto may require.

20.12. This Agreement may be executed in any number of counterparts, each of which when delivered will be deemed to be an original, for all purposes and will constitute one and the same instrument, binding on the parties, notwithstanding that all the parties are not signatories of the same counterpart.

20.13. In the event of a conflict arising between the interpretation of this Agreement and the Collaborative Research Agreement, the terms of this Agreement shall prevail.

IN WITNESS WHEREOF the parties hereto have hereunto executed this Agreement on the 15th day of February, 2001 but effective as of the Date of Commencement.

SIGNED FOR AND ON BEHALF of)
THE UNIVERSITY OF BRITISH COLUMBIA)
by its duly authorized officers:)
)
)
_____)
Authorized Signatory)
)
)
_____)
Authorized Signatory)

THE CORPORATE SEAL of)
XENON GENETICS INC)

was hereunto affixed in the presence of:)
)
) c/s
Authorized Signatory)
)
)
Authorized Signatory)

SCHEDULE "***A***"

DESCRIPTION OF "***TECHNOLOGY***"

UBC File #	Title	Inventors	Patents
94-061	Lipolipase Mutation 291, Implication for Coronary Artery Disease		U.S.:5,658,729 U.S.: SN 08/817,192 Can.: SN 2,202,477 EPO: 95 93 75 98.1 (Ger., Fr., U.K., Switz.)
91-003	Mutation in Human Lipoprotein Lipase Gene which causes Type 1 Hyperlipoproteinemia		Can.: SN 2,035,177
99-082	Recombinant Viruses Preparation and use thereof in Gene Therapy		U.S.: SN 08/737,954 FR: 94/06759 PCT: FR 95/00669 CIP: SN 09/713,268
00-039	Mutation 447		PCT: CA00/00762

000145

XENON

XENON GENETICS INC.
the Clinical Genomics Company™
3650 Gilmore Way
Burnaby, BC
Canada V5G 4W8
T 604.484.3300
F 604.484.3450
www.xenongenetics.com

September 27, 2002

Amsterdam Molecular Therapeutics B.V.
Meibergdreef 61
1105 BA
Amsterdam, The Netherlands

Attention: Dr Eric van der Aa, Ph.D.
VP Business Development

Dear Eric:

Re: Sublicense and Research Agreement dated June 18, 2001 between Xenon Genetics Inc. and Amsterdam Molecular Therapeutics B.V.
Research Project: "LPL Gene Therapy for LPL Deficiency"

This letter confirms our mutual agreement that the research project under the above-noted Sublicense and Research Agreement (the "Agreement") will continue for an additional year. We therefore confirm our agreement to the following Agreement amendments:

1. Capitalized Terms used in this letter agreement (hereinafter referred to as "Amendment #1") that are not otherwise defined herein shall have the meanings given to those terms in the Agreement except as amended by this Amendment #1.

2. Replace Section 1.2(e) with:

"Contract Period" shall mean August 1, 2000 to July 31, 2003.

3. Replace Section 2.3 with:

Xenon acknowledges receipt of payments totaling $600,000.00. Payments for the remaining $[**] shall be made by AMI to Xenon in the amounts and on the dates shown below:

Upon execution of this Amendment #1	*$*	*[**]*
October 15, 2002	*$*	*[**]*
January 15, 2003	*$*	*[**]*
April 15, 2003	*$*	*[**]*

---

4. Append Schedule A-1 to Appendix A of Annex 1

Schedule A-1 entitled "Statement of Work - Effective August 1, 2002" is attached.

5. Except as amended herein, the Agreement, and all other terms and conditions therein will remain in full force and effect, unamended.

Upon each parties' affixing of their respective signatures below, this Amendment will be effective as of August 1, 2002.

Please confirm your agreement to the above by having this document executed (in duplicate) on behalf of Amsterdam Molecular Therapeutics B.V., and return both copies to my attention. I will then make arrangements for Xenon's signature, and will return a fully-executed copy to you.

Please contact me at (604) 484-3304 or by e-mail at sfidai@xenongenetics.com if you have any questions.

Yours truly,

/s/ Shafique Fidai

Shafique Fidai
Associate Director, Business Development

Acknowledged and agreed to on behalf of
Amsterdam Molecular Therapeutics B.V.

/s/ Jan J.B. Boesen
Jan J.B. Boesen
Chief Executive Officer
Date: 01 Oct '02

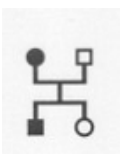
Acknowledged and agreed to on behalf of
Xenon Genetics, Inc.

/s/ Frank A. Holler
Frank A. Holler
President & CeO
Date: Oct 6/02

cc: Dr. Michael Hayden, Chief Scientific Officer, Xenon Genetics Inc.

Confidential Materials omitted and filed separately with the Securities and Exchange Commission. A total of 14 pages were omitted. [**]

XENON



XENON GENETICS INC.
a Clinical Genomics Company
3650 Gilmore Way
Burnaby, BC
Canada V5G4W8
T 604.484.3300
F 604.484.3450
www.xenongenetics.com

September 5, 2003

Amsterdam Molecular Therapeutics B.V.
Meibergdreef 61
1105 BA
Amsterdam, The Netherlands

Attention: Dr. Eric van der Aa, Ph.D.
VP Business Development

Dear Eric:

Re: Sublicense and Research Agreement dated June 18, 2001 between Xenon Genetics Inc. and Amsterdam Molecular Therapeutics B.V.as amended August 1, 2002
Research Project: "LPL Gene Therapy for LPL Deficiency"

This letter confirms our mutual agreement that the research project under the above-noted Sublicense and Research Agreement (the "Agreement"') will continue for an additional year. We therefore confirm our agreement to the following Agreement amendments:

1. Capitalized terms used in this letter agreement (hereinafter referred to as "Amendment #2") that are not otherwise defined herein shall have the meanings given to those terms in the Agreement except as amended by this Amendment #2.

2. Replace Section 1.2(e) with:

"Contract Period" shall mean August 1, 2000 to July 31, 2004.

3. Replace Section 2.3 with:

Payments totaling $[**] shall be made by AMT to Xenon in the amounts and on the dates shown below:

Upon execution of this Amendment #2	*$*	*[**]*
October 15, 2003	*$*	*[**]*
January 15, 2004	*$*	*[**]*
Apni 15, 2004	*$*	*[**]*

4. Append Schedule A-2 to Appendix A of Annex 1

Schedule A-2 entitled "LPL Gene Therapy tor Lipoprotein Lipase Deficiency - 2003-2004 Research Budget and Work Plan" is attached.

5. Except as amended herein, the Agreement, and all other terms and conditions therein will remain in full force and effect, unamended.

Upon each parties' affixing of their respective signatures below, this Amendment will be effective as of August 1, 2003.

Please confirm your agreement to the above by having this document executed (in duplicate) on behalf of Amsterdam Molecular Therapeutics B.V., and return both copies to my attention I will then make arrangements for Xenon's signature, and will return a fully-executed copy to you.

Please contact me at (604) 484-3308 or by e-mail at kcorraini@xenongenetics.com if you have any questions.

Yours truly,

Karen G. Corraini
General Counsel

Acknowledged and agreed to on behalf of **Amsterdam Molecular Therapeutics B.V.**

/s/ Jan J.B. Boesen
Jan J.B. Boesen
Chief Executive Officer
Date: 22 Sep. '03

Acknowledged and agreed to on behalf of **Xenon Genetics, Inc.**

/s/ Simon N. Pimstone
Simon N. Pimstone
President & COO
Date: 11 Sep. 2003

cc: Dr. Michael Hayden, Chief Scientific Officer, Xenon Genetics Inc.

SCHEDULE A-2

LPL GENE THERAPY FOR LIPOPROTEIN LIPASE DEFICIENCY:

2003-2004 Research Budget and Work Plan

XENON

XENON GENETICS INC.
the Clinical Genomics Company™
3650 Gilmore Way
Burnaby, BC
Canada V5G 4W8
T 604.484.3300
F 604.484.3450
www.xenongenetics.com

November 25, 2004

Amsterdam Molecular Therapeutics B.V.
Meibergdreef 61
1105 BA
Amsterdam, The Netherlands

Attention: Dr. Eric van der Aa, Ph.D.
VP Business Development

Dear Eric:

Re: Sublicense and Research Agreement dated June 18, 2001 between Xenon Genetics Inc. (now known as Xenon Pharmaceuticals Inc.) and Amsterdam Molecular Therapeutics B.V. as amended August 1, 2002 and August 1, 2003
Research Project: "LPL Gene Therapy for LPL Deficiency"

This letter confirms our mutual agreement that the research project under the above-noted Sublicense and Research Agreement (the "Agreement") will continue for an additional year. We therefore confirm our agreement to the following Agreement amendments:

1. Capitalized terms used in this letter agreement (hereinafter referred to as "**Amendment #3**") that are not otherwise defined herein shall have the meanings given to those terms in the Agreement except as amended by this Amendment #3.

2. Replace Section 1 2(e) with:

   "Contract Period" shall mean August 1, 2000 to July 31, 2005.

3. Replace Section 2.3 with:

   Payments totaling $[**] shall be made by AMT to Xenon in the amounts and on the dates shown below:

Upon execution of this Amendment #3	*$*	*[**]*
January 15, 2005	*$*	*[**]*
Apni 15, 2005	*$*	*[**]*

4. Append Schedule A 3 to Appendix A of Annex 1

   Schedule A-3 entitled "AAV1-LPL^{S447X} Gene Therapy for Lipoprotein Lipase Deficiency: 2004 - 2005 Work Plan and Budget" is attached.

5. Except as amended herein, the Agreement, and all other terms and conditions therein will remain in full force and effect, unamended.

Upon each parties' affixing of their respective signatures below, this Amendment will be effective as of August 1, 2004.

Please confirm your agreement to the above by having this document executed (in duplicate) on behalf of Amsterdam Molecular Therapeutics B.V., and return both copies to my attention. I will then make arrangements for Xenon's signature, and will return a fully-executed copy to you.

Please contact me at (604) 484-3308 or by e-mail at kcorraini@xenon-pharma.com if you have any questions.

Yours truly,

Karen G. Corraini
General Counsel

Acknowledged and agreed to on behalf of **Amsterdam Molecular Therapeutics B.V.**	Acknowledged and agreed to on behalf of **Xenon Pharmaceuticals Inc.**
/s/ Jan J.B. Boesen Jan J.B. Boesen Chief Executive Officer	/s/ Simon N. Pimstone Simon N. Pimstone President & CEO
Date: 26 November 2008	Date: 30 November 2004

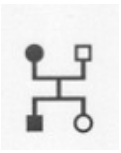
cc: Dr. Michael Hayden, Chief Scientific Officer, Xenon Pharmaceuticals Inc.

SCHEDULE A-3

AAV1-LPL^{S447X} Gene Therapy for
Lipoprotein Lipase Deficiency:

2004-2005 Work Plan and Budget

Confidential Materials omitted and filed separately with the Securities and Exchange Commission. A total of 14 pages were omitted. [**]



XENON
PHARMACEUTICALS INC.

3650 Gilmore Way
Burnaby, BC
Canada V5G 4W8

T 604.484.3300
F 604.484.3450

www.xenon-pharma.com

November 24, 2005

Amsterdam Molecular Therapeutics B.V.
Meibergdreef 61
1105 BA
Amsterdam, The Netherlands

Attention: Professor Sander van Deventer
Chief Scientific Officer

Dear Professor van Deventer

Re: Sublicense and Research Agreement dated June 18, 2001 between Xenon Genetics Inc. (now Xenon Pharmaceuticals Inc.) and Amsterdam Molecular Therapeutics B.V. as amended August 1, 2002, August 1, 2003 and August 1, 2004
Research Project: "LPL Gene Therapy for LPL Deficiency"

This letter confirms our mutual agreement that the research project under the above-noted Sublicense and Research Agreement (the "**Agreement**") will continue for an additional year. We therefore confirm our agreement to the following Agreement amendments:

1. Capitalized terms used in this letter agreement (hereinafter referred to as "**Amendment #4**") that are not otherwise defined herein shall have the meanings given to those terms in the Agreement except as amended by this Amendment #4.

2. Replace Section 1.2(e) with:

"Contract Period" shaft mean August 1, 2000 to July 31, 2006.

3. Replace Section 2.3 with:

Payments totaling $[**] shall be made by AMT to Xenon in the amounts and on the dates shown below:

Upon execution of this Amendment #4	*$*	*[**]*

XENON

January 15, 2006	*$*	*[**]*
April 15, 2006	*$*	*[**]*

4. Append Schedule A-4 to Appendix A of Annex 1

Schedule A-4 entitled "AAV 1-LPL^{S447X} Gene Therapy for Lipoprotein Lipase Deficiency: 2005-2006 Work Plan and Budget" is attached.

5. Except as amended herein, the Agreement, and all other terms and conditions therein will remain in full force and effect, unamended.

Upon each parties' affixing of their respective signatures below, this Amendment will be effective as of August 1, 2005.

Please confirm your agreement to the above by having this document executed (in duplicate) on behalf of Amsterdam Molecular Therapeutics B.V., and return both copies to my attention, I will then make arrangements for Xenon's signature, and will return a fully-executed copy to you.

Please contact me at (604) 484-3308 or by e-mail at kcorraini@xenon-pharma.com if you have any questions.

Yours truly,

Karen G. Corraini
General Counsel

Acknowledged and agreed to on behalf of
Amsterdam Molecular Therapeutics B.V.

Sander van Deventer
Chief Scientific Officer
Date: 29 Nov. 2005

Acknowledged and agreed to on behalf of
Xenon Pharmaceuticals Inc.

Simon N. Pimstone
President & CEO
Date:

cc: Dr. Michael Hayden, Chief Scientific Officer, Xenon Pharmaceuticals Inc.

SCHEDULE A-4

AAV1-LPL^{S447X} Gene Therapy for Lipoprotein Lipase Deficiency:

2005-2006 Work Plan and Budget

Confidential Materials omitted and filed separately with the Securities and Exchange Commission. A total of 25 pages were omitted. [**]

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XENON
PHARMACEUTICALS INC.

3650 Gilmore Way
Burnaby, BC
Canada V5G 4W8

T 604.484.3300
F 604.484.3450

www.xenon-pharma.com

Amsterdam Molecular Therapeutics B.V.
Meibergdreef 61
1105 BA
Amsterdam, The Netherlands

Attention: Jaap Twisk, PhD
Senior Scientist Preclinical Research

Dear Dr. Twisk:

Re: Sublicense and Research Agreement dated June 18, 2001 between Xenon Genetics Inc. (now Xenon Pharmaceuticals Inc.) and Amsterdam Molecular Therapeutics B.V. as amended August 1, 2002, August 1, 2003, August 1, 2004, and August 1, 2005
Research Project: "LPL Gene Therapy for LPL Deficiency"

This letter confirms our mutual agreement (i) to extend the Project under the above-noted Sublicense and Research Agreement (the **"Agreement"**) for an additional year, and (ii) to amend certain other provisions of the Agreement, as follows:

1. Capitalized terms used in this letter agreement (hereinafter referred to as **"Amendment #5"**) that are not otherwise defined herein shall have the meanings given to those terms in the Agreement except as amended by this Amendment #5.

2. Replace Section 1.2(e) with:

"Contract Period" shall mean August 1, 2000 to July 31, 2007.

3. Replace Section 2.3 with:

Payments totaling $[**] shall be made by AMT to Xenon in the amounts and on the dates shown below:

Upon execution of this Amendment #5	*$*	*[**]*
January 15, 2007	*$*	*[**]*
April 15, 2007	*$*	*[**]*

4. Add New Section 2.9:

Xenon confirms and agrees that, as part of the performance of the 2006-2007 Project, it will devote reasonable commercial efforts to ensuring that:

(a) UBC provides regular updates to AMT by email and/or teleconference;

(b) UBC discusses the progress of the Project with AMT on a quarterly basis, by teleconference, such teleconferences to include a discussion of results obtained and a consultation regarding next steps to be undertaken;

(c) UBC issues a progress report prior to such teleconference, upon AMT's prior written request, and.

(d) When either UBC or AMT determines that an in-person meeting between UBC and AMT is appropriate, such meeting(s) will be scheduled at a time and place to be mutually agreed to by UBC and AMT. AMT confirms and agrees to promptly reimburse UBC's out-of-pocket expenses, including airfare, hotels, meals and other travel costs, incurred to attend any such in-person meetings occurring outside of Vancouver, British Columbia.

5. Amend Section 12.1 by adding the following:

(a) The Parties hereto confirm that, depending on progress achieved in the Project, AMT reserves the right to terminate the Project prior to the end of the Contract Period or to request that Xenon renegotiate the Project's work plan and budget as set out in the appended Schedule A-5 and Section 2.3 as amended herein.

(b) In the event that AMT wishes to exercise its rights in subsection (a) above, AMT shall provide Xenon with ninety (90) days' prior written notice of such intent to terminate and/or request to amend the Project.

(c) In the event that AMT requests an amendment to the Project, the Parties agree to negotiate in good faith with the objective of agreeing upon said amendment within thirty (30) days following Xenon's receipt of AMT's written notice.

(d) In the event that the Parties are unable to reach an agreement on such amendment within that thirty (30) day period, the Project shall terminate in its entirety sixty (60) days thereafter.

(e) In the event of termination contemplated in subsection (b) or (d) above, AMT shall pay all costs and liabilities relating to the Project which have been incurred by Xenon and/or UBC as of the effective date of termination. For greater certainty, such costs and liabilities shall include all non-cancellable obligations including payments in lieu of reasonable notice for technicians, graduate students, and other staff assigned to the Project.

(f) For the avoidance of doubt, such termination will not release the Parties from any rights or obligations under the Agreement which would survive the termination or expiration of the Project.

6. Append updated Project description as Schedule A-5 to Appendix A of Annex 1

Updated Project description for the twelve-month period August 1,2006 to July 31, 2007, entitled "AAV1-LPL^{S447X} Gene Therapy for Lipoprotein Lipase Deficiency: 2006 - 2007 Work Plan and Budget" is attached.

7. In the event of a conflict between this Amendment #5 and Article 2 in the Agreement, this Amendment #5 shall prevail.

8. Except as amended herein, the Agreement, and all other terms and conditions therein will remain in full force and effect, unamended.

Upon each parties' affixing of their respective signatures below, this Amendment will be effective as of August 1, 2006.

Please confirm your agreement to the above by having this document executed on behalf of Amsterdam Molecular Therapeutics B.V., and fax back a fully-executed version of same to my attention. If AMT also requires

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an original hard copy of this Amendment, please let me know and we will arrange for such hard copies/duplicates to be executed and circulated.

Please contact me at (604) 484-3308 or by e-mail at kcorraini@xenon-pharma.com if you have any questions.

Yours truly,

/s/ Karen G. Corraini

Karen G. Corraini
General Counsel

Acknowledged and agreed to on behalf of
Amsterdam Molecular Therapeutics B.V.

/s/ Ronald Lorijn

Ronald Lorign
Chief Executive Officer

Acknowledged and agreed to on behalf of
Xenon Pharmaceuticals Inc.

/s/ Simon N. Pimstone

Simon N. Pimstone
President & CEO

cc: Dr. Michael Hayden, Chief Scientific Officer, Xenon Pharmaceuticals Inc.

SCHEDULE A-5

**AAV1-LPL^{S447X} Gene Therapy for
Lipoprotein Lipase Deficiency:**

2006-2007 Work Plan and Budget

Confidential Materials omitted and filed separately with the Securities and Exchange Commission. A total of 20 pages were omitted. [**]

XENON 000865

XENON
PHARMACEUTICALS INC.

3650 Gilmore Way
Burnaby, BC
Canada V5G 4W8

T 604.484.3300
F 604.484.3450

www.xenon-pharma.com

June 5, 2008

Amsterdam Molecular Therapeutics B.V.
Meibergdreef 61
1105 BA
Amsterdam, The Netherlands

Attention: Jaap Twisk, PhD
Senior Scientist Preclinical Research

Dear Dr. Twisk:

Re: Sublicense and Research Agreement dated June 18, 2001 between Xenon Genetics Inc. (now Xenon Pharmaceuticals Inc.) and Amsterdam Molecular Therapeutics B.V. as amended August 1, 2002, August 1, 2003, August 1, 2004, August 1, 2005 and August 1, 2006
Research Project: "LPL Gene Therapy for LPL Deficiency"

This letter confirms our mutual agreement to extend the Project under the above-noted Sublicense and Research Agreement (the "**Agreement**") for an additional year, as follows:

1. Capitalized terms used in this letter agreement (hereinafter referred to as "**Amendment #6**") that are not otherwise defined herein shall have the meanings given to those terms in the Agreement except as amended by this Amendment #6.

2. Replace Section 1.2(e) with:

"Contract Period" shall mean August 1, 2000 to June 30, 2008.

3. Replace Section 2.3 with:

Payments totaling $[**] shall be made by AMT to Xenon in the amounts and on the dates shown below:

Upon execution of this Amendment #6	*$*	*[**]*
Within five (5) business days following the date that CMMT and/or Xenon advises AMT that CMMT is ready to ship the LK mouse embryos to AMT (as contemplated under section 4.2.3 in the Project description attached)	*$[**]*	

For the avoidance of doubt, the parties confirm that i) shortly following the date that AMT receives the above-noted advisory, AMT and CMMT will confer to discuss the shipping arrangements for these embryos; (ii) CMMT will ship the LK mouse embryos to AMT using the method of shipment preferred by AMT; (iii) such shipment will take place on a date and time to be mutually agreed to between AMT and CMMT; and (iv) on or before the shipping date, AMT will have received the requisite permissions from the original owners of the mice (as contemplated under section 4.2.3 of the Project description attached), and will have provided CMMT with a copy of such permission for its records.

4. Append updated Project description as Schedule A-6 to Appendix A of Annex 1

Updated Project description for the eleven-month period August 1, 2007 to June 30, 2008, entitled "AAV1-LPL^{S447X} Gene Therapy for Lipoprotein Lipase Deficiency: 2007 - 2008 Work Plan and Budget" is attached.

5. In the event of a conflict between this Amendment #6 and Article 2 in the Agreement, this Amendment #6 shall prevail.

6. Except as amended herein, the Agreement, and all other terms and conditions therein will remain in full force and effect, unamended.

Upon each parties' affixing of their respective signatures below, this Amendment will be effective as of August 1, 2007.

Please confirm your agreement to the above by having this document executed on behalf of Amsterdam Molecular Therapeutics B.V., and fax or scan/email back a fully-executed version of same to my attention. If AMT also requires an original hard copy of this Amendment, please let me know and we will arrange for such hard copies/duplicates to be executed and circulated.

Please contact me at (604) 484-3308 or by e-mail at kcorraini@xenon-pharma.com if you have any questions.

Yours truly,

Karen G. Corraini
General Counsel

Acknowledged and agreed to on behalf of **Amsterdam Molecular Therapeutics B.V.**

Ronald Lorijn
Chief Executive Officer

Acknowledged and agreed to on behalf of **Xenon Genetics, Inc.**

Simon N. Pimstone
President & CEO

cc: Dr. Michael Hayden, Chief Scientific Officer, Xenon Pharmaceuticals Inc.

AAV1-LPL^{S447X} Gene Therapy for Lipoprotein Lipase Deficiency:

2007-2008 Work Plan and Budget

Confidential Materials omitted and filed separately with the Securities and Exchange Commission. A total of 21 pages were omitted. [**]

uniQure N.V.

2014 Share Incentive Plan

1. Purpose

The purpose of this 2014 Share Incentive Plan (the "**Plan**") of uniQure N.V., a public limited company incorporated under the laws of the Netherlands (the "**Company**"), is to advance the interests of the Company's shareholders by enhancing the Company's ability to attract, retain and motivate persons who are expected to make important contributions to the Company and by providing such persons with equity ownership opportunities and performance-based incentives that are intended to better align the interests of such persons with those of the Company's shareholders. Except where the context otherwise requires, the term "**Company**" shall include any of the Company's present or future parent or subsidiary corporations as defined in Sections 424(e) or (f) of the U.S. Internal Revenue Code of 1986, as amended, and any regulations thereunder (the "**Code**") and any other business venture (including, without limitation, joint venture or limited liability company) in which the Company has a controlling interest, as determined by the Supervisory Board of the Company (the "**Supervisory Board**").

2. Eligibility

All of the Company's employees, managing directors and supervisory directors, as well as consultants and advisors to the Company (as such terms are defined and interpreted for purposes of Form S-8 under the Securities Act of 1933, as amended (the "**Securities Act**"), or any successor form) are eligible to be granted Awards under the Plan. Eligibility to participate in the Plan shall be determined at the sole discretion of the Supervisory Board. Each person who is granted an Award under the Plan is deemed a "**Participant**." "**Award**" means Options (as defined in Section 5), SARs (as defined in Section 6), Restricted Shares (as defined in Section 7), Restricted Share Units (as defined in Section 7) and Other Share-Based Awards (as defined in Section 8).

3. Administration and Delegation

(a) Administration by Supervisory Board. The Plan will be administered by the Supervisory Board. The Supervisory Board shall have authority to grant Awards and to adopt, amend and repeal such administrative rules, guidelines and practices relating to the Plan as it shall deem advisable. The Supervisory Board may construe and interpret the terms of the Plan and any Award agreements entered into under the Plan. The Supervisory Board may correct any defect, supply any omission or reconcile any inconsistency in the Plan or any Award in the manner and to the extent it shall deem expedient and it shall be the sole and final judge of such expediency. All decisions by the Supervisory Board shall be made in the Supervisory Board's sole discretion and shall be final and binding on all persons having or claiming any interest in the Plan or in any Award.

(b) Appointment of Committees. To the extent permitted by applicable law, the Supervisory Board may delegate any or all of its powers under the Plan to one or more committees or subcommittees of the Supervisory Board (a "**Committee**"). All references in the Plan to the "**Supervisory Board**" shall mean the Supervisory Board or a Committee of the Supervisory Board to the extent that the Supervisory Board's powers or authority under the Plan have been delegated to such Committee.

4. Shares Available for Awards

(a) Number of Shares; Share Counting.

(1) Authorized Number of Shares. Subject to adjustment under Section 9, Awards may be made under the Plan for up to such number of ordinary shares (€[0.0·] par value per share) of the Company (the "**Ordinary Shares**") as is equal to [·].

(2) Share Counting. For purposes of counting the number of shares available for the grant of Awards under the Plan:

(A) all Ordinary Shares covered by SARs shall be counted against the number of shares available for the grant of Awards under the Plan; provided, however, that (i) SARs that may be settled only in cash shall not be so counted and (ii) if the Company grants a SAR in tandem with an Option for the same number of Ordinary Shares and provides that only one such Award may be exercised (a "**Tandem SAR**"), only the shares covered by the Option, and not the shares covered by the Tandem SAR, shall be so counted, and the expiration of one in connection with the other's exercise will not restore shares to the Plan;

(B) if any Award (i) expires or is terminated, surrendered or canceled without having been fully exercised or is forfeited in whole or in part (including as the result of Ordinary Shares subject to such Award being repurchased by the Company at the original issuance price pursuant to a contractual repurchase right) or (ii) results in any Ordinary Shares not being issued (including as a result of a SAR that was settleable either in cash or in shares actually being settled in cash), the unused Ordinary Shares covered by such Award shall again be available for the grant of Awards; provided, however, that (1) in the case of Incentive Share Options, the foregoing shall be subject to any limitations under the Code, (2) in the case of the exercise of a SAR, the number of shares counted against the shares available under the Plan shall be the full number of shares subject to the SAR multiplied by the percentage of the SAR actually exercised, regardless of the number of shares actually used to settle such SAR upon exercise and (3) the shares covered by a Tandem SAR shall not again become available for grant upon the expiration or termination of such Tandem SAR; and

(C) Ordinary Shares delivered (either by actual delivery, attestation, or net exercise) to the Company by a Participant to (i) purchase Ordinary Shares upon the exercise of an Award or (ii) satisfy tax withholding obligations with respect to Options and SARs (including shares retained from the Option or SAR creating the tax obligation) shall not be added back to the number of shares available for the future grant of Awards.

(b) Substitute Awards. In connection with a merger or consolidation of an entity with the Company or the acquisition by the Company of property or stock of an entity, the

Supervisory Board may grant Awards in substitution for any options or other share or share-based awards granted by such entity or an affiliate thereof. Substitute Awards may be granted on such terms as the Supervisory Board deems appropriate in the circumstances, notwithstanding any limitations on Awards contained in the Plan. Substitute Awards shall not count against the overall share limit set forth in Section 4(a), except as may be required by reason of Section 422 and related provisions of the Code.

5. Share Options

(a) General. The Supervisory Board may grant options to purchase Ordinary Shares (each, an "**Option**") and determine the number of Ordinary Shares to be covered by each Option, the exercise price of each Option and the conditions and limitations applicable to the exercise of each Option, including conditions relating to applicable securities laws, as it considers necessary or advisable.

(b) Incentive Share Options. An Option that the Supervisory Board intends to be an "incentive stock option" as defined in Section 422 of the Code (an "**Incentive Share Option**") shall only be granted to employees of uniQure N.V., any of uniQure N.V.'s present or future parent or subsidiary corporations as defined in Sections 424(e) or (f) of the Code, and any other entities the employees of which are eligible to receive Incentive Share Options under the Code, and shall be subject to and shall be construed consistently with the requirements of Section 422 of the Code. An Option that is not intended to be an Incentive Share Option shall be designated a "**Share Option**." The Company shall have no liability to a Participant, or any other party, if an Option (or any part thereof) that is intended to be an Incentive Share Option is not an Incentive Share Option or if the Company converts an Incentive Share Option to a Share Option. Awards with respect to a maximum of [2,000,000] Ordinary Shares may be granted in the form of Incentive Share Options under the Plan.

(c) Exercise Price. The Supervisory Board shall establish the exercise price of each Option and specify the exercise price in the applicable Option agreement which may be less than, equal to, or greater than the Fair Market Value per Ordinary Share on the date the Option is granted; provided, however, (i) that the exercise price of (A) any Option intended to be an Incentive Share Option and (B) any Share Option granted to a Participant who is subject to taxation in the United States for U.S. federal income tax purposes shall be not less than 100% of the Fair Market Value per Ordinary Share on the date the Option is granted and provided further, that if the Supervisory Board approves the grant of an Option with an exercise price to be determined on a future date, in the case of (A) and (B), the exercise price shall be not less than 100% of the Fair Market Value on such future date. For purposes of the Plan, the fair market value per Ordinary Share, shall be determined by (or in a manner approved by) the Supervisory Board ("**Fair Market Value**") and (ii) in no event shall the exercise price of any Option be less than the nominal value per Ordinary Share.

(d) Duration of Options. Each Option shall be exercisable at such times and subject to such terms and conditions as the Supervisory Board may specify in the applicable option agreement; provided, however, that no Option will be granted with a term in excess of 10 years.

(e) Exercise of Options. Options may be exercised by delivery to the Company of a notice of exercise in a form (which may be electronic) approved by the Company, together with payment in full (in the manner specified in Section 5(f)) of the exercise price for the number of shares for which the Option is exercised. Ordinary Shares subject to the Option will be delivered by the Company as soon as practicable following exercise.

(f) Payment Upon Exercise. Ordinary Shares purchased upon the exercise of an Option granted under the Plan shall be paid for as follows:

(1) By wire transfer, in cash or by check, payable to the order of the Company;

(2) except as may otherwise be provided in the applicable Option agreement or approved by the Supervisory Board, in its sole discretion, by (i) delivery of an irrevocable and unconditional undertaking by a creditworthy broker to deliver promptly to the Company sufficient funds to pay the exercise price and any required tax withholding or (ii) delivery by the Participant to the Company of a copy of irrevocable and unconditional instructions to a creditworthy broker to deliver promptly to the Company cash or a check sufficient to pay the exercise price and any required tax withholding;

(3) to the extent provided for in the applicable Option agreement or approved by the Supervisory Board, in its sole discretion, by delivery (either by actual delivery or attestation) of Ordinary Shares owned by the Participant valued at their Fair Market Value, provided (i) such method of payment is then permitted under applicable law, (ii) such Ordinary Shares, if acquired directly from the Company, were owned by the Participant for such minimum period of time, if any, as may be established by the Supervisory Board in its discretion and (iii) such Ordinary Shares are not subject to any repurchase, forfeiture, unfulfilled vesting or other similar requirements;

(4) to the extent provided for in the applicable Share Option agreement or approved by the Supervisory Board in its sole discretion, by delivery of a notice of "net exercise" to the Company, as a result of which the Participant would receive (i) the number of shares underlying the portion of the Option being exercised, less (ii) such number of shares as is equal to (A) the aggregate exercise price for the portion of the Option being exercised divided by (B) the Fair Market Value on the date of exercise;

(5) to the extent permitted by applicable law and provided for in the applicable Option agreement or approved by the Supervisory Board, in its sole discretion, by payment of such other lawful consideration as the Supervisory Board may determine; or

(6) by any combination of the above permitted forms of payment.

6. Share Appreciation Rights

(a) General. The Supervisory Board may grant Awards consisting of share appreciation rights ("**SARs**") entitling the holder, upon exercise, to receive an amount of Ordinary Shares or cash or a combination thereof (such form to be determined by the Supervisory Board) determined by reference to appreciation, from and after the date of grant, in

the Fair Market Value of an Ordinary Share over the measurement price established pursuant to Section 6(b). The date as of which such appreciation is determined shall be the exercise date.

(b) Measurement Price. The Supervisory Board shall establish the measurement price of each SAR and specify it in the applicable SAR agreement. The measurement price shall not be less than 100% of the Fair Market Value on the date the SAR is granted; provided that if the Supervisory Board approves the grant of a SAR effective as of a future date, the measurement price shall be not less than 100% of the Fair Market Value on such future date.

(c) Duration of SARs. Each SAR shall be exercisable at such times and subject to such terms and conditions as the Supervisory Board may specify in the applicable SAR agreement; provided, however, that no SAR will be granted with a term in excess of 10 years.

(d) Exercise of SARs. SARs may be exercised by delivery to the Company of a notice of exercise in a form (which may be electronic) approved by the Company, together with any other documents required by the Supervisory Board.

7. Restricted Shares; Restricted Share Units

(a) General. The Supervisory Board may grant Awards entitling recipients to acquire Ordinary Shares ("**Restricted Shares**"), subject to the right of the Company to repurchase all or part of such shares at their issue price or other stated or formula price (or to require forfeiture of such shares if issued at no cost) from the recipient in the event that conditions specified by the Supervisory Board in the applicable Award are not satisfied prior to the end of the applicable restriction period or periods established by the Supervisory Board for such Award. The Supervisory Board may also grant Awards entitling the recipient to receive Ordinary Shares or cash to be delivered at the time such Award vests ("**Restricted Share Units**") (Restricted Shares and Restricted Share Units are each referred to herein as a "**Restricted Share Award**").

(b) Terms and Conditions for All Restricted Share Awards. The Supervisory Board shall determine the terms and conditions of a Restricted Share Award, including the conditions for vesting and repurchase (or forfeiture) and the issue price, if any.

(c) Additional Provisions Relating to Restricted Shares.

(1) Dividends. Unless otherwise provided in the applicable Award agreement, any dividends (whether paid in cash or shares) declared and paid by the Company with respect to shares of Restricted Shares ("**Accrued Dividends**") shall be paid to the Participant only if and when such shares become free from the restrictions on transferability and forfeitability that apply to such shares. Each payment of Accrued Dividends will be made no later than the end of the calendar year in which the dividends are paid to shareholders of that class of shares or, if later, the 15th day of the third month following the lapsing of the restrictions on transferability and the forfeitability provisions applicable to the underlying shares of Restricted Share.

(d) Additional Provisions Relating to Restricted Share Units.

(1) Settlement. Upon the vesting of and/or lapsing of any other restrictions (i.e., settlement) with respect to each Restricted Share Unit, the Participant shall be entitled to receive from the Company the number of shares of Ordinary Shares set forth in the applicable Award agreement or (if so provided in the applicable Award agreement) an amount of cash equal to the Fair Market Value of one of such number of Ordinary Shares. The Supervisory Board may, in its discretion, provide that settlement of Restricted Share Units shall be deferred, on a mandatory basis or at the election of the Participant in a manner that complies with Section 409A of the Code.

(2) Voting Rights. A Participant shall have no voting rights with respect to any Restricted Share Units.

(3) Dividend Equivalents. The Award agreement for Restricted Share Units may provide Participants with the right to receive an amount equal to any dividends or other distributions declared and paid on an equal number of outstanding Ordinary Shares ("**Dividend Equivalents**"). Dividend Equivalents may be paid currently or credited to an account for the Participant, may be settled in cash and/or Ordinary Shares and may be subject to the same restrictions on transfer and forfeitability as the Restricted Share Units with respect to which paid, in each case to the extent provided in the Award agreement.

8. Other Share-Based Awards

(a) General. Other Awards of Ordinary Shares, and other Awards that are valued in whole or in part by reference to, or are otherwise based on, Ordinary Shares or other property, may be granted hereunder to Participants ("**Other Share-Based Awards**"). Such Other Share-Based Awards shall also be available as a form of payment in the settlement of other Awards granted under the Plan or as payment in lieu of compensation to which a Participant is otherwise entitled. Other Share-Based Awards may be paid in Ordinary Shares or cash, as the Supervisory Board shall determine.

(b) Terms and Conditions. Subject to the provisions of the Plan, the Supervisory Board shall determine the terms and conditions of each Other Share-Based Award, including any purchase price applicable thereto.

9. Adjustments for Changes in Ordinary Shares and Certain Other Events

(a) Changes in Capitalization. In the event of any share split, share consolidation, share dividend, recapitalization, combination of shares, reclassification of shares, spin-off or other similar change in capitalization or event, or any dividend or distribution to holders of Ordinary Shares other than an ordinary cash dividend, (i) the number and class of securities available under the Plan, (ii) the share counting rules set forth in Section 4(a), (2) the number and class of securities and exercise price per share of each outstanding Option, (iv) the share and per-share provisions and the measurement price of each outstanding SAR, (v) the number of shares subject to and the repurchase price per share subject to each outstanding Restricted Share Award and (vi) the share and per-share-related provisions and the purchase price, if any, of each outstanding Other Share-Based Award, shall be equitably adjusted by the Company (or substituted Awards may be made, if applicable) in the manner determined by the Supervisory

Board. Without limiting the generality of the foregoing, in the event the Company effects a split of the Ordinary Shares by means of a share dividend and the exercise price of and the number of shares subject to an outstanding Option are adjusted as of the date of the distribution of the dividend (rather than as of the record date for such dividend), then an optionee who exercises an Option between the record date and the distribution date for such share dividend shall be entitled to receive, on the distribution date, the share dividend with respect to the Ordinary Shares acquired upon such Option exercise, notwithstanding the fact that such shares were not outstanding as of the close of business on the record date for such share dividend.

(b) Reorganization Events.

(1) Definition. A "**Reorganization Event**" shall mean: (a) any merger or consolidation of the Company with or into another entity as a result of which all of the Ordinary Shares of the Company are converted into or exchanged for the right to receive cash, securities or other property or are cancelled, (b) any transfer or disposition of all of the Ordinary Shares of the Company for cash, securities or other property pursuant to a share exchange or other transaction or (c) any liquidation or dissolution of the Company.

(2) Consequences of a Reorganization Event on Awards Other than Restricted Shares.

(A) In connection with a Reorganization Event, the Supervisory Board may take any one or more of the following actions as to all or any (or any portion of) outstanding Awards other than Restricted Shares on such terms as the Supervisory Board determines (except to the extent specifically provided otherwise in an applicable Award agreement or another agreement between the Company and the Participant): (i) provide that such Awards shall be assumed, or substantially equivalent Awards shall be substituted, by the acquiring or succeeding corporation (or an affiliate thereof), (ii) upon written notice to a Participant, provide that all of the Participant's unexercised and/or unvested Awards will terminate immediately prior to the consummation of such Reorganization Event unless exercised by the Participant (to the extent then exercisable) within a specified period following the date of such notice, (iii) 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, (iv) in the event of a Reorganization Event under the terms of which holders of Ordinary Shares will receive upon consummation thereof a cash payment for each share surrendered in the Reorganization Event (the "**Acquisition Price**"), make or provide for a cash payment to Participants with respect to each Award held by a Participant equal to (A) the number of shares 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 (B) the excess, if any, of (I) the Acquisition Price over (II) the exercise, measurement or purchase price of such Award and any applicable tax withholdings, in exchange for the termination of such Award, (v) provide that, in connection with a liquidation or dissolution of the Company, Awards shall convert into the right to receive liquidation proceeds (if applicable, net of the exercise, measurement or purchase price thereof and any applicable tax withholdings) and (vi) any combination of the foregoing. In taking any of the actions permitted under this Section 9(b)(2), the Supervisory Board shall not be obligated by the Plan to treat all Awards, all Awards held by a Participant, or all Awards of the same type, identically.

(B) Notwithstanding the terms of Section 9(b)(2)(A), in the case of outstanding Restricted Share Units that are subject to Section 409A of the Code: (i) if the applicable Restricted Share Unit agreement provides that the Restricted Share Units shall be settled upon a "change in control event" within the meaning of U.S. Treasury Regulation Section 1.409A-3(i)(5)(i), and the Reorganization Event constitutes such a "change in control event", then no assumption or substitution shall be permitted pursuant to Section 9(b)(2)(A)(i) and the Restricted Share Units shall instead be settled in accordance with the terms of the applicable Restricted Share Unit agreement; and (ii) the Supervisory Board may only undertake the actions set forth in clauses (iii), (iv) or (v) of Section 9(b)(2)(A) if the Reorganization Event constitutes a "change in control event" as defined under U.S. Treasury Regulation Section 1.409A-3(i)(5)(i) and such action is permitted or required by Section 409A of the Code; if the Reorganization Event is not a "change in control event" as so defined or such action is not permitted or required by Section 409A of the Code, and the acquiring or succeeding corporation does not assume or substitute the Restricted Share Units pursuant to clause (i) of Section 9(b)(2)(A), then the unvested Restricted Share Units shall terminate immediately prior to the consummation of the Reorganization Event without any payment in exchange therefor.

(C) For purposes of Section 9(b)(2)(A)(i), an Award (other than Restricted Shares) shall be considered assumed if, following consummation of the Reorganization Event, such Award confers the right to purchase or receive pursuant to the terms of such Award, for each Ordinary Share subject to the Award immediately prior to the consummation of the Reorganization Event, the consideration (whether cash, securities or other property) received as a result of the Reorganization Event by holders of Ordinary Shares for each Ordinary Share held immediately prior to the consummation of the Reorganization Event (and if holders were offered a choice of consideration, the type of consideration chosen by the holders of a majority of the outstanding Ordinary Shares); provided, however, that if the consideration received as a result of the Reorganization Event is not solely ordinary shares or common stock of the acquiring or succeeding corporation (or an affiliate thereof), the Company may, with the consent of the acquiring or succeeding corporation, provide for the consideration to be received upon the exercise or settlement of the Award to consist solely of such number of ordinary shares or common stock of the acquiring or succeeding corporation (or an affiliate thereof) that the Supervisory Board determined to be equivalent in value (as of the date of such determination or another date specified by the Supervisory Board) to the per share consideration received by holders of outstanding Ordinary Shares as a result of the Reorganization Event.

(3) Consequences of a Reorganization Event on Restricted Shares. Upon the occurrence of a Reorganization Event other than a liquidation or dissolution of the Company, the repurchase and other rights of the Company with respect to outstanding Restricted Shares shall inure to the benefit of the Company's successor and shall, unless the Supervisory Board determines otherwise, apply to the cash, securities or other property which the Ordinary Shares were converted into or exchanged for pursuant to such Reorganization Event in the same manner and to the same extent as they applied to such Restricted Shares; provided, however, that the Supervisory Board may provide for termination or deemed satisfaction of such repurchase or other rights under the instrument evidencing any Restricted Shares or any other agreement between a Participant and the Company, either initially or by amendment. Upon the occurrence of a Reorganization Event involving the liquidation or dissolution of the Company, except to the extent specifically provided to the contrary in the instrument evidencing any Restricted Shares or

any other agreement between a Participant and the Company, all restrictions and conditions on all Restricted Shares then outstanding shall automatically be deemed terminated or satisfied.

10. General Provisions Applicable to Awards

(a) Transferability of Awards. Awards shall not be sold, assigned, transferred, pledged or otherwise encumbered by the person to whom they are granted, either voluntarily or by operation of law, except by will or the laws of descent and distribution applicable to such Participant or, other than in the case of an Incentive Share Option, pursuant to a qualified domestic relations order, and, during the life of the Participant, shall be exercisable only by the Participant; provided, however, that the Supervisory Board may permit or provide in an Award for the gratuitous transfer of the Award by the Participant to or for the benefit of any immediate family member, family trust or other entity established for the benefit of the Participant and/or an immediate family member thereof if the Company would be eligible to use a Form S-8 under the Securities Act for the registration of the sale of the Ordinary Shares subject to such Award to such proposed transferee; provided further, that the Company shall not be required to recognize any such permitted transfer until such time as such permitted transferee shall, as a condition to such transfer, deliver to the Company a written instrument in form and substance satisfactory to the Company confirming that such

transferee shall be bound by all of the terms and conditions of the Award. References to a Participant, to the extent relevant in the context, shall include references to authorized transferees. For the avoidance of doubt, nothing contained in this Section 10(a) shall be deemed to restrict a transfer to the Company.

(b) Documentation. Each Award shall be evidenced in such form (written, electronic or otherwise) as the Supervisory Board shall determine. Each Award may contain terms and conditions in addition to those set forth in the Plan.

(c) Supervisory Board Discretion. Except as otherwise provided by the Plan, each Award may be made alone or in addition or in relation to any other Award. The terms of each Award need not be identical, and the Supervisory Board need not treat Participants uniformly.

(d) Termination of Status. The Supervisory Board shall determine the effect on an Award of the disability, death, termination or other cessation of employment, authorized leave of absence or other change in the employment or other status of a Participant and the extent to which, and the period during which, the Participant, or the Participant's legal representative, conservator, guardian or Designated Beneficiary, may exercise rights under the Award. "**Designated Beneficiary**" means (i) the beneficiary designated, in a manner determined by the Supervisory Board, by a Participant to receive amounts due or exercise rights of the Participant in the event of the Participant's death or (ii) in the absence of an effective designation by a Participant, the Participant's estate.

(e) Withholding. The Participant must satisfy all applicable Dutch, United States and other applicable national, federal, state, and local or other income, national insurance, social and employment tax withholding obligations before the Company will deliver or otherwise recognize ownership of Ordinary Shares under an Award. The Company may decide to satisfy the withholding obligations through additional withholding on salary or wages. If the Company elects not to or cannot withhold from other compensation, the Participant must pay the Company

the full amount, if any, required for withholding or have a broker tender to the Company cash equal to the withholding obligations. Payment of withholding obligations is due before the Company will issue any shares on exercise, vesting or release from forfeiture of an Award or at the same time as payment of the exercise or purchase price, unless the Company determines otherwise. If provided for in an Award or approved by the Supervisory Board in its sole discretion, a Participant may satisfy such tax obligations in whole or in part by delivery (either by actual delivery or attestation) of Ordinary Shares, including shares retained from the Award creating the tax obligation, valued at their Fair Market Value; provided, however, except as otherwise provided by the Supervisory Board, that the total tax withholding where shares are being used to satisfy such tax obligations cannot exceed the Company's minimum statutory withholding obligations (based on minimum statutory withholding rates for Dutch, United States and other applicable national, federal and state tax purposes, including payroll taxes, that are applicable to such supplemental taxable income). Shares used to satisfy tax withholding requirements cannot be subject to any repurchase, forfeiture, unfulfilled vesting or other similar requirements.

(f) Amendment of Award. The Supervisory Board may amend, modify or terminate any outstanding Award, including but not limited to, substituting therefor another Award of the same or a different type, changing the date of exercise or realization, and converting an Incentive Share Option to a Share Option. The Participant's consent to such action shall be required unless (i) the Supervisory Board determines that the action, taking into account any related action, does not materially and adversely affect the Participant's rights under the Plan or (ii) the change is permitted under Section 9.

(g) Conditions on Delivery of Ordinary Shares. The Company will not be obligated to deliver any Ordinary Shares pursuant to the Plan or to remove restrictions from shares previously issued or delivered under the Plan until (i) all conditions of the Award have been met or removed to the satisfaction of the Company, (ii) in the opinion of the Company's counsel, all other legal matters in connection with the issuance and delivery of such shares have been satisfied, including any applicable securities laws and regulations and any applicable stock exchange or stock market rules and regulations, and (iii) the Participant has executed and delivered to the Company such representations or agreements as the Company may consider appropriate to satisfy the requirements of any applicable laws, rules or regulations.

(h) Acceleration. The Supervisory Board may at any time provide that any Award shall become immediately exercisable in whole or in part, free of some or all restrictions or conditions, or otherwise realizable in whole or in part, as the case may be.

11. Miscellaneous

(a) No Right To Employment or Other Status. No person shall have any claim or right to be granted an Award by virtue of the adoption of the Plan, and the grant of an Award shall not be construed as giving a Participant the right to continued employment or any other relationship with the Company. The Company expressly reserves the right at any time to dismiss or otherwise terminate its relationship with a Participant free from any liability or claim under the Plan, except as expressly provided in the applicable Award. This Plan will not be considered a part of any employment agreement in force between the Participant and the Company and/or a

group company. The grant of an Award does not qualify as an employment condition and shall not be included in the calculation of any severance payment or any other payments in connection with the Participant's employment agreement or the termination thereof. The granting of an Award or the vesting thereof does not in any way affect the scope or level of the Participant's pension rights, pension entitlements and/or of any other entitlements vis-a-vis the Company and/or a group company. The granting of an Award is at the sole discretion of the Supervisory Board and does not entitle the Participant to any future Awards.

(b) No Rights As Shareholder. Subject to the provisions of the applicable Award, no Participant or Designated Beneficiary shall have any rights as a shareholder with respect to any Ordinary Shares to be distributed with respect to an Award until becoming the record holder of such shares.

(c) Effective Date and Term of Plan. The Plan shall become effective on the date the Plan is approved by the Company's shareholders (the "**Effective Date**"). No Awards shall be granted under the Plan after the expiration of 10 years from the Effective Date, but Awards previously granted may extend beyond that date.

(d) Amendment of Plan. The Supervisory Board may amend, suspend or terminate the Plan or any portion thereof at any time provided that (i) to the extent required by Section 162(m) of the Code, no Award granted to a Participant that is intended to comply with Section 162(m) of the Code after the date of such amendment shall become exercisable, realizable or vested, as applicable to such Award, unless and until the Company's shareholders approve such amendment in the manner required by Section 162(m) of the Code; and (ii) no amendment that would require shareholder approval under the rules of the NASDAQ Stock Market may be made effective unless and until the Company's shareholders approve such amendment. In addition, if at any time the approval of

the Company's shareholders is required as to any other modification or amendment under Section 422 of the Code or any successor provision with respect to Incentive Share Options, the Supervisory Board may not effect such modification or amendment without such approval. Unless otherwise specified in the amendment, any amendment to the Plan adopted in accordance with this Section 11(d) shall apply to, and be binding on the holders of, all Awards outstanding under the Plan at the time the amendment is adopted, provided the Supervisory Board determines that such amendment, taking into account any related action, does not materially and adversely affect the rights of Participants under the Plan. No Award shall be made that is conditioned upon shareholder approval of any amendment to the Plan unless the Award provides that (i) it will terminate or be forfeited if shareholder approval of such amendment is not obtained within no more than 12 months from the date of grant and (2) it may not be exercised or settled (or otherwise result in the issuance of Ordinary Shares) prior to such shareholder approval.

(e) Authorization of Sub-Plans. The Supervisory Board may from time to time establish one or more sub-plans under the Plan for purposes of satisfying applicable securities, tax or other laws of various jurisdictions. The Supervisory Board shall establish such sub-plans by adopting supplements to the Plan containing (i) such limitations on the Supervisory Board's discretion under the Plan as the Supervisory Board deems necessary or desirable or (ii) such additional terms and conditions not otherwise inconsistent with the Plan as the Supervisory Board shall deem necessary or desirable. All supplements adopted by the Supervisory Board

shall be deemed to be part of the Plan, but each supplement shall apply only to Participants within the affected jurisdiction and the Company shall not be required to provide copies of any supplement to Participants in any jurisdiction which is not the subject of such supplement.

(f) Compliance with Section 409A of the Code. Except as provided in individual Award agreements initially or by amendment, if and to the extent (i) any portion of any payment, compensation or other benefit provided to a Participant pursuant to the Plan in connection with his or her employment termination constitutes "nonqualified deferred compensation" within the meaning of Section 409A of the Code and (ii) the Participant is a specified employee as defined in Section 409A(a)(2)(B)(i) of the Code, in each case as determined by the Company in accordance with its procedures, by which determinations the Participant (through accepting the Award) agrees that he or she is bound, such portion of the payment, compensation or other benefit shall not be paid before the day that is six months plus one day after the date of "separation from service" (as determined under Section 409A of the Code) (the "**New Payment Date**"), except as Section 409A of the Code may then permit. The aggregate of any payments that otherwise would have been paid to the Participant during the period between the date of separation from service and the New Payment Date shall be paid to the Participant in a lump sum on such New Payment Date, and any remaining payments will be paid on their original schedule.

The Company makes no representations or warranty and shall have no liability to the Participant or any other person if any provisions of or payments, compensation or other benefits under the Plan are determined to constitute nonqualified deferred compensation subject to Section 409A of the Code but do not to satisfy the conditions of that section.

(g) Limitations on Liability. Notwithstanding any other provisions of the Plan, no individual acting as a supervisory director, managing director, employee or agent of the Company will be liable to any Participant, former Participant, spouse, beneficiary, or any other person for any claim, loss, liability, or expense incurred in connection with the Plan, nor will such individual be personally liable with respect to the Plan because of any contract or other instrument he or she executes in his or her capacity as a supervisory director, managing director, employee or agent of the Company. The Company will indemnify and hold harmless each supervisory director, managing director, employee or agent of the Company to whom any duty or power relating to the administration or interpretation of the Plan has been or will be delegated, against any cost or expense (including attorneys' fees) or liability (including any sum paid in settlement of a claim with the Supervisory Board's approval) arising out of any act or omission to act concerning the Plan unless arising out of such person's own fraud or bad faith.

(h) Data Protection. The Participant hereby fully consents to the processing and transfer of all relevant data in the context of the administration of this Plan and the Award Agreement. The Participant shall keep the Company fully informed of any changes in the relevant data.

(i) Insider Trading. The Participant shall comply with the Company's policies on insider trading.

(j) Governing Law. The provisions of the Plan and all Awards made hereunder shall be governed by and interpreted in accordance with the laws of the Netherlands, excluding

choice-of-law principles of the law of such state that would require the application of the laws of a jurisdiction other than the Netherlands. Any disputes arising out of or in connection with the Plan shall, to the extent permitted by law, be submitted exclusively to the competent court of Amsterdam, the Netherlands.

Exhibit 10.23

uniQure N.V.

Incentive Share Option Agreement
Granted Under 2014 Share Incentive Plan

1. Grant of Option.

This agreement evidences the grant by uniQure N.V., a public limited company incorporated under the laws of the Netherlands (the "**Company**"), on [·] (the "Grant Date") to [·] (the "**Participant**"), of an option to purchase, in whole or in part, on the terms provided herein and in the Company's 2014 Share Incentive Plan (the "**Plan**"), a total of [·] ordinary shares, [€0.0·] par value per share, of the Company ("**Ordinary Shares**") at [·] per share.(1) Unless earlier terminated, this option shall expire at 17:00, Central European time, on [·] (the "**Final Exercise Date**").(2)

It is intended that the option evidenced by this agreement shall be an incentive stock option as defined in Section 422 of the U.S. Internal Revenue Code of 1986, as amended, and any regulations promulgated thereunder (the "**Code**"). Except as otherwise indicated by the context, the term "Participant", as used in this option, shall be deemed to include any person who acquires the right to exercise this option validly under its terms.

2. Vesting Schedule.

This option will become exercisable ("**vest**") as to 25% of the original number of Ordinary Shares on the first anniversary of the Grant Date and as to an additional 6.25% of the original number of Ordinary Shares at the end of each successive three-month period following the first anniversary of the Grant Date until the fourth anniversary of the Grant Date.

The right of exercise shall be cumulative so that to the extent the option is not exercised in any period to the maximum extent permissible it shall continue to be exercisable, in whole or in part, with respect to all Ordinary Shares for which it is vested until the earlier of the Final Exercise Date or the termination of this option under Section 3 hereof or the Plan.

3. Exercise of Option.

(a) Form of Exercise. Each election to exercise this option shall be in writing, signed by the Participant, and received by the Company at its principal office, accompanied by this agreement, or by such other method as shall be approved by the Company, in each case together with payment in full in the manner provided in the Plan. The Participant may purchase less than

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(1) This must be at least 100% of the fair market value of the Ordinary Shares on the date of grant (or 110% in the case of a Participant that owns more than 10% of the total combined voting power of all classes of stock of the Company or its parent or subsidiary (a "10% Shareholder")) for the option to qualify as an incentive stock option (an "ISO") under Section 422 of the Code.

(2) The Final Exercise Date must be no more than 10 years (5 years in the case of a 10% Shareholder) from the date of grant for the option to qualify as an ISO. The correct approach to calculate the final exercise date is to use the day immediately prior to the date ten years out from the date of the stock option award grant (5 years in the case of a 10% stockholder). For example, an award granted to someone on October 1, 2001 would expire on September 30, 2011 (not on October 1, 2011).

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the number of shares covered hereby, provided that no partial exercise of this option may be for any fractional share or for fewer than ten whole shares.

(b) Continuous Relationship with the Company Required. Except as otherwise provided in this Section 3, this option may not be exercised unless the Participant, at the time he or she exercises this option, is, and has been at all times since the Grant Date, an employee, officer or director of, or consultant or advisor (as such terms are defined for purposes of Form S-8 under the Securities Act of 1933, as amended) to, the Company or any parent or subsidiary of the Company as defined in Section 424(e) or (f) of the Code (an "**Eligible Participant**").

(c) Termination of Relationship with the Company. If the Participant ceases to be an Eligible Participant for any reason, then, except as provided in paragraphs (d) and (e) below, the right to exercise this option shall terminate three months after such cessation (but in no event after the Final Exercise Date), provided that this option shall be exercisable only to the extent that the Participant was entitled to exercise this option on the date of such cessation. Notwithstanding the foregoing, if the Participant, prior to the Final Exercise Date, violates the non-competition or confidentiality provisions of any employment contract, confidentiality and nondisclosure agreement or other agreement between the Participant and the Company, the right to exercise this option shall terminate immediately upon written notice to the Participant from the Company describing such violation.

(d) Exercise Period Upon Death or Disability. If the Participant dies or becomes disabled (within the meaning of Section 22(e)(3) of the Code) prior to the Final Exercise Date while he or she is an Eligible Participant and the Company has not terminated such relationship for "cause" as specified in paragraph (e) below, this option shall be exercisable, within the period of one year following the date of death or disability of the Participant, by the Participant (or in the case of death by an authorized transferee), provided that this option shall be exercisable only to the extent that this option was exercisable by the Participant on the date of his or her death or disability, and further provided that this option shall not be exercisable after the Final Exercise Date.

(e) Termination for Cause. If, prior to the Final Exercise Date, the Participant's employment is terminated by the Company for Cause (as defined below), the right to exercise this option shall terminate immediately upon the effective date of such termination of employment. If, prior to the Final Exercise Date, the Participant is given notice by the Company of the termination of his or her employment by the Company for Cause, and the effective date of such employment termination is subsequent to the date of delivery of such notice, the right to exercise this option shall be suspended from the time of the delivery of such notice until the earlier of (i) such time as it is determined or otherwise agreed that the Participant's employment shall not be terminated for Cause as provided in such notice or (ii) the effective date of such termination of employment (in which case the right to exercise this option shall, pursuant to the preceding sentence, terminate upon the effective date of such termination of employment). If the Participant is party to an employment or severance agreement with the Company that contains a definition of "cause" for termination of employment, "Cause" shall have the meaning ascribed to such term in such agreement. Otherwise, "Cause" shall mean willful misconduct by the Participant or willful failure by the Participant to perform his or her responsibilities to the Company (including, without limitation, breach by the Participant of any provision of any

employment, consulting, advisory, nondisclosure, non-competition or other similar agreement between the Participant and the Company), as determined by the Company, which determination shall be conclusive. The Participant's employment shall be considered to have been terminated for Cause if the Company determines, within 30 days after the Participant's resignation, that termination for Cause was warranted.

4. Tax Matters.

(a) Withholding. No Ordinary Shares will be issued pursuant to the exercise of this option unless and until the Participant pays to the Company, or makes provision satisfactory to the Company for payment of, any national, federal, state and local or other income, national insurance, social and employment taxes required by law to be withheld in respect of this option.

(b) Disqualifying Disposition. If the Participant disposes of Ordinary Shares acquired upon exercise of this option within two years from the Grant Date or one year after such Ordinary Shares were acquired pursuant to exercise of this option, the Participant shall notify the Company in writing of such disposition.

5. Transfer Restrictions.

(a) This option may not be sold, assigned, transferred, pledged or otherwise encumbered by the Participant, either voluntarily or by operation of law, except by will or the laws of descent and distribution, and, during the lifetime of the Participant, this option shall be exercisable only by the Participant.

(b) The Participant agrees that he or she will not transfer any Ordinary Shares issued pursuant to the exercise of this option unless the transferee, as a condition to such transfer, delivers to the Company a written instrument confirming that such transferee shall be bound by all of the terms and conditions of Section 4; provided that such a written confirmation shall not be required with respect to Section 4 the completion of the lock-up period in connection with the Company's initial underwritten public offering.

6. Provisions of the Plan.

This option is subject to the provisions of the Plan (including the provisions relating to amendments to the Plan), a copy of which is furnished to the Participant with this option.

7. Nature of the Grant.

In accepting the option, the Participant acknowledges that:

(a) the Plan is established voluntarily by the Company, it provides for certain criteria in order to be eligible to receive an award, it is restricted in time, it is discretionary in nature and it may be modified, amended, suspended or terminated by the Company at any time, unless otherwise provided in the Plan and this agreement;

(b) the grant of the option is voluntary and occasional and does not create any contractual or other right to receive future grants of options, or benefits in lieu of options, even if options have been granted repeatedly in the past;

(c) all decisions with respect to future option grants, if any, will be at the sole discretion of the Supervisory Board;

(d) the Participant is voluntarily participating in the Plan;

(e) the options are an extraordinary item that does not constitute compensation of any kind for services of any kind rendered to the Company, and which is outside the scope of the Participant's employment or consultancy agreement of his or her corporate mandate, if any;

(f) the options are not part of normal or expected compensation or salary for any purposes, including, but not limited to, calculation of any severance, resignation, termination, redundancy, end of service payments, bonuses, long-service awards, pension, retirement or welfare benefits or similar payments and in no event should be considered as compensation for, or relating in any way, to past services for the Company;

(g) in the event that the Participant is not an employee of uniQure N.V., the options and the Participant's participation in the Plan will not be interpreted to form an employment or service contract or relationship with the Company;

(h) the future value of the underlying Ordinary Shares is unknown and cannot be predicted with certainty; if the Participant's options never vest, the Participant will not be able to exercise the options; and

(i) in consideration of the options, no claim or entitlement to compensation or damages shall arise from termination of the options or from any decrease in value of the options or Ordinary Shares acquired upon exercise of the options resulting from termination of the Participant's employment, consultancy or corporate mandate by or with the Company (for any reason whatsoever and whether or not in breach of contract or local laws) and the Participant irrevocably releases the Company from any such claim that may arise.

8. Data Privacy.

The Participant hereby explicitly and unambiguously consents to the collection, use and transfer, in electronic or other form, of his or her personal data as described in this agreement by and among, as applicable, his or her employer or contracting party and the Company for the exclusive purpose of implementing, administering and managing his or her participation in the Plan.

The Participant understands that the Company holds certain personal information about him or her, including, but not limited to, his or her name, home address and telephone number, work location and phone number, date of birth, hire date, details of all options or any other entitlement to Ordinary Shares awarded, cancelled, exercised, vested, unvested or outstanding in the Participant's favor, for the purpose of implementing, administering and managing the Plan ("Personal Data"). The Participant understands that Personal Data may be transferred to any

third parties assisting in the implementation, administration and management of the Plan, that these recipients may be located in the Participant's country or elsewhere, and that the recipient's country may have different data privacy laws and protections than the Participant's country. The Participant understands that he or she may request a list with the names and addresses of any potential recipients of the Personal Data by contacting his or her local human resources representative. The Participant authorizes the recipients to receive, possess, use, retain and transfer the Personal Data, in electronic or other form, for the purposes of implementing, administering and managing his or her participation in the Plan, including any requisite transfer of such Personal Data as may be required to a broker or other third party with whom the Participant may elect to deposit any Ordinary Shares acquired upon exercise of the options. The Participant understands that Personal Data will be held only as long as is necessary to implement, administer and manage his or her participation in the Plan. The Participant understands that he or she may, at any time, view Personal Data, request additional information about the storage and processing of Personal Data, require any necessary amendments to Personal Data or refuse or withdraw the consents herein, in any case without cost, by contacting in writing his or her local human resources representative. The Participant understands, however, that refusing or withdrawing his or her consent may affect his or her ability to participate in the Plan. For more information on the consequences of Participant's refusal to consent or withdrawal of consent, the Participant understands that he or she may contact his or her local human resources representative.

The Company has caused this option to be executed by its duly authorized officer.

UNIQURE N.V.

By: _____
Name: _____
Title: _____

PARTICIPANT'S ACCEPTANCE

The undersigned hereby accepts the foregoing option and agrees to the terms and conditions thereof. The undersigned hereby acknowledges receipt of a copy of the Company's 2014 Share Incentive Plan.

PARTICIPANT

_____
Name: _____

Address:

uniQure N.V.

Share Option Agreement
Granted Under 2014 Share Incentive Plan

1. Grant of Option.

This agreement evidences the grant by uniQure N.V., a public limited company incorporated under the laws of the Netherlands (the "**Company**"), on [·] (the "**Grant Date**") to [·] (the "Participant"), of an option to purchase, in whole or in part, on the terms provided herein and in the Company's 2014 Share Incentive Plan (the "**Plan**"), a total of [·] ordinary shares, [€0.0·] par value per share, of the Company ("**Ordinary Shares**") at [·] per share. Unless earlier terminated, this option shall expire at 17:00, Central European time, on [·] (the "**Final Exercise Date**").

It is intended that the option evidenced by this agreement shall not be an incentive stock option as defined in Section 422 of the U.S. Internal Revenue Code of 1986, as amended, and any regulations promulgated thereunder (the "**Code**"). Except as otherwise indicated by the context, the term "Participant", as used in this option, shall be deemed to include any person who acquires the right to exercise this option validly under its terms.

2. Vesting Schedule.

This option will become exercisable ("**vest**") as to 25% of the original number of Ordinary Shares on the first anniversary of the Grant Date and as to an additional 6.25% of the original number of Ordinary Shares at the end of each successive three-month period following the first anniversary of the Grant Date until the fourth anniversary of the Grant Date.

The right of exercise shall be cumulative so that to the extent the option is not exercised in any period to the maximum extent permissible it shall continue to be exercisable, in whole or in part, with respect to all Ordinary Shares for which it is vested until the earlier of the Final Exercise Date or the termination of this option under Section 3 hereof or the Plan.

3. Exercise of Option.

(a) Form of Exercise. Each election to exercise this option shall be in writing, signed by the Participant, and received by the Company at its principal office, accompanied by this agreement, or by such other method as shall be approved by the Company, in each case together with payment in full in the manner provided in the Plan. The Participant may purchase less than the number of shares covered hereby, provided that no partial exercise of this option may be for any fractional share or for fewer than ten whole shares.

(b) Continuous Relationship with the Company Required. Except as otherwise provided in this Section 3, this option may not be exercised unless the Participant, at the time he or she exercises this option, is, and has been at all times since the Grant Date, an employee, officer or a director of, or consultant or advisor (as such terms are defined for purposes of Form S-8 under the Securities Act of 1933, as amended) to, the Company or any other entity the

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employees, officers, directors, consultants, or advisors of which are eligible to receive option grants under the Plan (an "Eligible Participant").

(c) Termination of Relationship with the Company. If the Participant ceases to be an Eligible Participant for any reason, then, except as provided in paragraphs (d) and (e) below, the right to exercise this option shall terminate three months after such cessation (but in no event after the Final Exercise Date), provided that this option shall be exercisable only to the extent that the Participant was entitled to exercise this option on the date of such cessation. Notwithstanding the foregoing, if the Participant, prior to the Final Exercise Date, violates the non-competition or confidentiality provisions of any employment contract, confidentiality and nondisclosure agreement or other agreement between the Participant and the Company, the right to exercise this option shall terminate immediately upon written notice to the Participant from the Company describing such violation.

(d) Exercise Period Upon Death or Disability. If the Participant dies or becomes disabled (within the meaning of Section 22(e)(3) of the Code) prior to the Final Exercise Date while he or she is an Eligible Participant and the Company has not terminated such relationship for "cause" as specified in paragraph (e) below, this option shall be exercisable, within the period of one year following the date of death or disability of the Participant, by the Participant (or in the case of death by an authorized transferee), provided that this option shall be exercisable only to the extent that this option was exercisable by the Participant on the date of his or her death or disability, and further provided that this option shall not be exercisable after the Final Exercise Date.

(e) Termination for Cause. If, prior to the Final Exercise Date, the Participant's employment or other relationship with the Company is terminated by the Company for Cause (as defined below), the right to exercise this option shall terminate immediately upon the effective date of such termination of employment or other relationship. If, prior to the Final Exercise Date, the Participant is given notice by the Company of the termination of his or her employment or other relationship by the Company for Cause, and the effective date of such employment or other termination is subsequent to the date of the delivery of such notice, the right to exercise this option shall be suspended from the time of the delivery of such notice until the earlier of (i) such time as it is determined or otherwise agreed that the Participant's employment or other relationship shall not be terminated for Cause as provided in such notice or (ii) the effective date of such termination of employment or other relationship (in which case the right to exercise this option shall, pursuant to the preceding sentence, terminate immediately upon the effective date of such termination of employment or other relationship). If the Participant is party to an employment, consulting or severance agreement with the Company that contains a definition of "cause" for termination of employment or other relationship, "Cause" shall have the meaning ascribed to such term in such agreement. Otherwise, "Cause" shall mean willful misconduct by the Participant or willful failure by the Participant to perform his or her responsibilities to the Company (including, without limitation, breach by the Participant of any provision of any employment, consulting, advisory, nondisclosure, non-competition or other similar agreement between the Participant and the Company), as determined by the Company, which determination shall be conclusive. The Participant's employment or other relationship shall be considered to have been terminated for "Cause" if the Company determines, within 30 days after the Participant's resignation, that termination for Cause was warranted.

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4. Withholding.

No Ordinary Shares will be issued pursuant to the exercise of this option unless and until the Participant pays to the Company, or makes provision satisfactory to the Company for payment of, any national, federal, state and local or other income, national insurance, social and employment taxes required by law to be withheld in respect of this option.

5. Transfer Restrictions.

(a) This option may not be sold, assigned, transferred, pledged or otherwise encumbered by the Participant, either voluntarily or by operation of law, except by will or the laws of descent and distribution relevant to the Participant, and, during the lifetime of the Participant, this option shall be exercisable only by the Participant.

(b) The Participant agrees that he or she will not transfer any Ordinary Shares issued pursuant to the exercise of this option unless the transferee, as a condition to such transfer, delivers to the Company a written instrument confirming that such transferee shall be bound by all of the terms and conditions of Section 4; provided that such a written confirmation shall not be required with respect to Section 4 after the completion of the lock-up period in connection with the Company's initial underwritten public offering.

6. Nature of the Grant.

In accepting the option, the Participant acknowledges that:

(a) the Plan is established voluntarily by the Company, it provides for certain criteria in order to be eligible to receive an award, it is restricted in time, it is discretionary in nature and it may be modified, amended, suspended or terminated by the Company at any time, unless otherwise provided in the Plan and this agreement;

(b) the grant of the option is voluntary and occasional and does not create any contractual or other right to receive future grants of options, or benefits in lieu of options, even if options have been granted repeatedly in the past;

(c) all decisions with respect to future option grants, if any, will be at the sole discretion of the Supervisory Board;

(d) the Participant is voluntarily participating in the Plan;

(e) the options are an extraordinary item that does not constitute compensation of any kind for services of any kind rendered to the Company, and which is outside the scope of the Participant's employment or consultancy agreement of his or her corporate mandate, if any;

(f) the options are not part of normal or expected compensation or salary for any purposes, including, but not limited to, calculation of any severance, resignation, termination, redundancy, end of service payments, bonuses, long-service awards, pension, retirement or welfare benefits or similar payments and in no event should be considered as compensation for, or relating in any way, to past services for the Company;

(g) in the event that the Participant is not an employee of uniQure N.V., the options and the Participant's participation in the Plan will not be interpreted to form an employment or service contract or relationship with the Company;

(h) the future value of the underlying Ordinary Shares is unknown and cannot be predicted with certainty; if the Participant's options never vest, the Participant will not be able to exercise the options; and

(i) in consideration of the options, no claim or entitlement to compensation or damages shall arise from termination of the options or from any decrease in value of the options or Ordinary Shares acquired upon exercise of the options resulting from termination of the Participant's employment, consultancy or corporate mandate by or with the Company (for any reason whatsoever and whether or not in breach of contract or local laws) and the Participant irrevocably releases the Company from any such claim that may arise.

7. Data Privacy.

The Participant hereby explicitly and unambiguously consents to the collection, use and transfer, in electronic or other form, of his or her personal data as described in this agreement by and among, as applicable, his or her employer or contracting party and the Company for the exclusive purpose of implementing, administering and managing his or her participation in the Plan.

The Participant understands that the Company holds certain personal information about him or her, including, but not limited to, his or her name, home address and telephone number, work location and phone number, date of birth, hire date, details of all options or any other entitlement to Ordinary Shares awarded, cancelled, exercised, vested, unvested or outstanding in the Participant's favor, for the purpose of implementing, administering and managing the Plan ("Personal Data"). The Participant understands that Personal Data may be transferred to any third parties assisting in the implementation, administration and management of the Plan, that these recipients may be located in the Participant's country or elsewhere, and that the recipient's country may have different data privacy laws and protections than the Participant's country. The Participant understands that he or she may request a list with the names and addresses of any potential recipients of the Personal Data by contacting his or her local human resources representative. The Participant authorizes the recipients to receive, possess, use, retain and transfer the Personal Data, in electronic or other form, for the purposes of implementing, administering and managing his or her participation in the Plan, including any requisite transfer of such Personal Data as may be required to a broker or other third party with whom the Participant may elect to deposit any Ordinary Shares acquired upon exercise of the options. The Participant understands that Personal Data will be held only as long as is necessary to implement, administer and manage his or her participation in the Plan. The Participant understands that he or she may, at any time, view Personal Data, request additional information about the storage and processing of Personal Data, require any necessary amendments to Personal Data or refuse or withdraw the consents herein, in any case without cost, by contacting in writing his or her local human resources representative. The Participant understands, however, that refusing or withdrawing his or her consent may affect his or her ability to participate in the Plan. For more information on the consequences of Participant's refusal to consent or withdrawal of consent, the

Participant understands that he or she may contact his or her local human resources representative.

8. Provisions of the Plan.

This option is subject to the provisions of the Plan (including the provisions relating to amendments to the Plan), a copy of which is furnished to the Participant with this option.

The Company has caused this option to be executed by its duly authorized officer.

UNIQURE N.V.

By: _____
Name: _____
Title: _____

PARTICIPANT'S ACCEPTANCE

The undersigned hereby accepts the foregoing option and agrees to the terms and conditions thereof. The undersigned hereby acknowledges receipt of a copy of the Company's 2014 Share Incentive Plan.

PARTICIPANT

_____
Name: _____

Address:

Exhibit 10.32

Confidential Materials omitted and filed separately with the
Securities and Exchange Commission. Double asterisks denote omissions.

Execution Copy
CONFIDENTIAL

COLLABORATION AND LICENSE AGREEMENT

BY AND BETWEEN

4D MOLECULAR THERAPEUTICS, LLC

AND

UNIQURE BIOPHARMA B.V.

JANUARY 17, 2014

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COLLABORATION AND LICENSE AGREEMENT

This Collaboration and License Agreement (this "Agreement") is entered into as of January 17, 2014 (the "Effective Date"), by and between 4D Molecular Therapeutics, LLC, a limited liability company organized and existing under the laws of the State of Delaware and having a principal office located at 19 Rima Court, Danville, CA 94526 ("4DMT"), and uniQure biopharma B.V., a corporation organized and existing under the laws of The Netherlands and having a principal office located at Meibergdreef 61, 1105 BA Amsterdam, The Netherlands ("uniQure").

INTRODUCTION

1. 4DMT is a biopharmaceutical company focused on discovering and developing novel adeno-associated viral vectors for delivery of nucleic acids to target cells.

2. uniQure is a biopharmaceutical company focused on the research, development, manufacturing and marketing of gene therapy based biopharmaceutical products.

3. 4DMT and uniQure desire to conduct a research collaboration to identify improved AAV Capsid Variants (as defined below).

4. As of even date herewith, uniQure B.V., an Affiliate of uniQure, has executed and delivered to 4DMT a commitment letter in the form set forth in Exhibit A.

5. uniQure desires to receive from 4DMT exclusive rights under 4DMT's intellectual property rights to research (subject to 4DMT's retained rights to conduct research under the Research Program), Develop, manufacture and Commercialize Selected Capsid Variants, Royalty Bearing Compounds and Royalty Bearing Products in the Field (each as defined below) pursuant to this Agreement.

NOW, THEREFORE, in consideration of the mutual covenants contained herein, and other good and valuable consideration, the receipt of which is hereby acknowledged, 4DMT and uniQure agree as follows effective as of the Effective Date:

ARTICLE I

DEFINITIONS

Unless specifically set forth to the contrary herein, the following terms, whether used in the singular or plural, shall have the respective meanings set forth below:

1.1 "4DMT AAV Capsid Variant". 4DMT Capsid Variant means any AAV Capsid Variant that does not carry a Gene Therapy Construct contained in a Royalty Bearing Compound or Royalty Bearing Product.

1.2 "4DMT AAV Capsid Variant Library". 4DMT AAV Capsid Variant Library means any AAV Capsid Variant Library constructed by or licensed to 4DMT, including all AAV Capsid Variant Libraries provided to 4DMT pursuant to the UCB Agreements.

1.3 "4DMT Intellectual Property". 4DMT Intellectual Property means the 4DMT Know-How and the 4DMT Patent Rights.

1.4 "4DMT Know-How". 4DMT Know-How means Know-How that is (a) Controlled by 4DMT or its Affiliates as of the Effective Date or during the Research Term, and (b) necessary or useful to conduct the Research Program or to research, Develop, make and have made, use or Commercialize the relevant Selected Capsid Variant, Royalty Bearing Compound or Royalty Bearing Product. 4DMT Know-How includes Core 4DMT Know-How but does not include Joint Know-How.

1.5 "4DMT Patent Right". 4DMT Patent Right means any Patent Right Controlled by 4DMT or its Affiliates as of the Effective Date or during the Term that Covers 4DMT Know-How. Schedule 1.5 lists the 4DMT Patent Rights existing as of the Effective Date. 4DMT Patent Rights include Core

4DMT Patent Rights but do not include Joint Patent Rights.

1.6 "AAV". AAV means adeno-associated virus.

1.7 "AAV Capsid Variant". AAV Capsid Variant means an AAV capsid that is modified as compared to the wild type sequence.

1.8 "AAV Capsid Variant Library". AAV Capsid Variant Library means a collection of variant AAV capsid open reading frames inserted into an AAV genome in a manner that renders such variants genome replication-competent with the appropriate helper virus functions and capable of being selected and evolved to optimize their ability to deliver nucleic acid sequences to human or animal cells.

1.9 "Accounting Standards". Accounting Standards means, with respect to uniQure and its Affiliates, International Financial Reporting Standards ("IFRS") or, to the extent applicable, generally accepted accounting principles as practiced in the United States ("GAAP"), and with respect to 4DMT and its Affiliates, GAAP, in each case as they exist from time to time, consistently applied.

1.10 "Affiliate". Affiliate means, with respect to a Party, any entity that directly or indirectly controls, is controlled by, or is under common control with such Party. As used in this definition, the term "control" means the possession, directly or indirectly, of the power to direct or cause the direction of the management and policies of an entity, whether through ownership of voting securities, by contract or otherwise. For purposes of this definition, "control" shall be presumed to exist if one of the following conditions are met: (a) in the case of corporate entities, direct or indirect ownership of more than fifty percent (50%) of the stock or shares having the right to vote for the election of directors, and (b) in the case of non-corporate entities, direct or indirect ownership of more than fifty percent (50%) of the equity interest with the power to direct the management and policies of such non-corporate entities.

1.11 "Animal POC". Animal POC means demonstration of safety and a Pre-agreed level of therapeutic efficacy, including a change in the levels of a Pre-agreed disease relevant biomarker in some cases as a substitute for therapeutic efficacy, in a Pre-agreed animal model.

1.12 "Business Day". Business Day means a day that is not a Saturday, Sunday or a day on which banking institutions in New York, New York, USA or Amsterdam, The Netherlands are authorized by Law to remain closed.

1.13 "Calendar Quarter". Calendar Quarter means the respective periods of three (3) consecutive calendar months ending on March 31, June 30, September 30 and December 31; provided, however, that the first Calendar Quarter hereunder shall commence on the Effective Date and the final Calendar Quarter hereunder shall end on the effective date of termination or expiration of this Agreement.

1.14 "Calendar Year". Calendar Year means each successive period of twelve (12) months commencing on January 1 and ending on December 31; provided, however, that the first Calendar Year hereunder shall commence on the Effective Date and the final Calendar Quarter hereunder shall end on the effective date of termination or expiration of this Agreement.

1.15 "Candidate Success Criteria". Candidate Success Criteria means the criteria that an AAV Capsid Variant identified through a Research Selection Process (or any Research Compound containing such AAV Capsid Variant) must meet before it progresses to the next stage of the Research Program, as determined and approved by the JRSC, and as further described in Section 3.3(a).

1.16 "CEO". CEO means the Chief Executive Officer of a Party or, if there is no Chief Executive Officer of a Party, the Board Chairperson or senior-most executive officer or equivalent of such Party.

1.17 "Clinical Trial(s)". Clinical Trial(s) means a Phase I Study, a Phase II clinical study, a Pivotal Study or a Phase III Study.

1.18 "Clinical POC". Clinical POC means demonstration of safety and a Pre-agreed level of therapeutic efficacy, including a change in the levels of a Pre-agreed disease relevant biomarker in some cases as a substitute for therapeutic efficacy, in a Pre-agreed number of human patients.

1.19 "Commercially Reasonable Efforts". Commercially Reasonable Efforts means, with respect to a Party, the efforts required in order to carry out a task in a diligent and sustained manner without undue interruption or delay, which level is at least commensurate with the level of effort that a similarly situated Third Party would devote to a product of similar market potential and having similar commercial and scientific advantages and disadvantages resulting from its own research efforts or to which it has rights, taking into account its safety and efficacy, regulatory status, the competitiveness of the marketplace, its proprietary position, pricing, reimbursement, launching strategy and other market-specific factors, and all other relevant factors.

1.20 "Commercialization" or "Commercialize". Commercialization or Commercialize means any activity directed to obtaining pricing or reimbursement approvals, marketing, promoting, distributing, importing, exporting, offering to sell or selling a product, or to have any such activity performed. When used as a verb, "Commercialize" means to engage in Commercialization.

1.21 "Compound". Compound means an AAV Capsid Variant carrying a Gene Therapy Construct.

1.22 "Confidential Information". Confidential Information means any and all information and data, including all uniQure Know-How, 4DMT Know-How and Joint Know-How, and all other scientific, pre-clinical, clinical, regulatory, manufacturing, marketing, financial and commercial information or data, whether communicated in writing or orally or by any other method, which is provided by one Party to the other Party in connection with this Agreement or the Prior Confidentiality Agreement. All Core uniQure Know-How shall be considered the Confidential Information of uniQure, with respect to which: (a) uniQure shall be considered the disclosing Party, (b) 4DMT shall be considered the receiving Party, and (c) clauses (b) and (e) of Section 8.2 shall not apply. All Core 4DMT Know-How shall be considered the Confidential Information of 4DMT, with respect to which: (i) 4DMT shall be considered the disclosing Party, (ii) uniQure shall be considered the receiving Party, and (iii) clauses (b) and (e) of Section 8.2 shall not apply.

1.23 "Control". Control means, with respect to any item of or right under Patent Rights or Know-How, the possession (whether by ownership or license, other than a license pursuant to this Agreement) of the ability of a Party or, as applicable, its Affiliate (subject to Section 12.7), to grant

access to, or a license or sublicense of, such items or right as provided for herein without violating the terms of any agreement or other arrangement with any Third Party existing at the time such Party would be required hereunder to grant the other Party such access or license or sublicense.

1.24 "Core 4DMT Intellectual Property". Core 4DMT Intellectual Property means Core 4DMT Know-How and Core 4DMT Patent Rights.

1.25 "Core 4DMT Know-How". Core 4DMT Know-How means any Know-How [**].

1.26 "Core 4DMT Patent Right". Core 4DMT Patent Right means any Patent Right that Covers the Core 4DMT Know-How.

1.27 "Core uniQure Intellectual Property". Core uniQure Intellectual Property means Core uniQure Know-How and Core uniQure Patent Rights.

1.28 "Core uniQure Know-How". Core uniQure Know-How means any Know-How [**].

1.29 "Core uniQure Patent Right". Core uniQure Patent Right means any Patent Right that Covers the Core uniQure Know-How.

1.30 "Cover", "Covering" or "Covered". Cover, Covering or Covered means, with respect to a product, technology, process or method that, in the absence of ownership of or a license granted under a Valid Claim, the manufacture, use, offer for sale, sale or importation of such product or the practice of such technology, process or method would infringe such Valid Claim (or, in the case of a Valid Claim that has not yet issued, would infringe such Valid Claim if it were to issue).

1.31 "Default". Default means with respect to a Party that (a) any representation or warranty of such Party set forth herein shall have been untrue in any material respect when made or (b) such Party shall have failed to perform any material obligation set forth in this Agreement.

1.32 "Delivery Success Criteria". Delivery Success Criteria means the following criteria that determines whether an AAV Capsid Variant demonstrates improved delivery or function of a Gene Therapy Construct: [**].

1.33 "Development" or "Develop". Development or Develop means pre-clinical and clinical drug development activities, including: test method development and stability testing, toxicology, formulation, process development, manufacturing scale-up, development-stage manufacturing, quality assurance/quality control procedure development and performance with respect to clinical materials, statistical analysis and report writing and clinical studies, regulatory affairs, and all other pre-Regulatory Approval activities. When used as a verb, "Develop" means to engage in Development.

1.34 "EMA". EMA means the European Medicines Agency, or any successor agency.

1.35 "European Union" or "EU". European Union or EU means the countries that are members of the European Union, as redefined from time to time.

1.36 "FDA" or "Food and Drug Administration". FDA or Food and Drug Administration means the United States Food and Drug Administration, or any successor agency.

1.37 "Field". Field means the delivery of Gene Therapy Constructs to cells in (a) the central nervous system ("CNS") or (b) the liver, in each case where such delivery is for the purpose of effecting expression of the applicable RNA or amino acid sequence in the targeted cells and is potentially useful for the diagnosis, treatment, palliation or prevention of a disease or medical condition in humans or animals, irrespective of the administration site or mode of administration (*e.g.*, intravenous, direct injection, subcutaneous or intrathecal) of the Compound used to effect delivery. For clarity, intravenous administration of any Compound targeted to cells in other organs (*i.e.*, not specifically targeted to liver or CNS tissues), including for treatment of neoplastic and eye disorders, is excluded from the Field.

1.38 "First Commercial Sale". First Commercial Sale means, with respect to any Royalty Bearing Product and a country, the first sale for end use or consumption of such Royalty Bearing Product in such country after all required approvals, including Regulatory Approval, have been granted by the Regulatory Authority of such country. For clarity, sales for

test marketing, sampling and promotional uses, clinical trials purposes or compassionate use shall not constitute a First Commercial Sale.

1.39 "FTE". FTE means [**] hours of work devoted to or in support directly of the Research Program that is carried out by one or more qualified scientific or technical employees of 4DMT or its Affiliates, measured in accordance with 4DMT's normal time allocation practices from time to time. Overtime, and work on weekends, holidays and the like, shall not be counted with any multiplier (*e.g.*, time-and-a-half or double time) toward the number of hours that are used to calculate the FTE contribution. The portion of an FTE billable for one (1) individual during a Calendar Quarter shall be determined by dividing the number of hours worked directly by said individual on the Research Program during such Calendar Quarter by [**] hours.

1.40 "FTE Costs". FTE Costs means, for any Calendar Quarter, the number of FTEs multiplied by the FTE Rate.

1.41 "FTE Rate". FTE Rate means the amount for each FTE as set forth in Schedule 1.41.

1.42 "Fully Burdened Manufacturing Cost". Fully Burdened Manufacturing Cost means, as applicable to a Royalty Bearing Product, the cost of manufacturing such Royalty Bearing Product, which is equal to the sum of (a) for such Royalty Bearing Product (or components thereof), the costs of all direct material, direct labor and allocable manufacturing overhead consumed, provided, or procured by a Party, in each case for the manufacture of such Royalty Bearing Product, and (b) for such Royalty Bearing Product (or components thereof) made by a Third Party, the out-of-pocket costs paid to such Third Party by a Party; in each case (a) and (b) to the extent such costs are incurred by a Party or its Affiliates and to the extent such costs are reasonably allocable to the manufacture of such Royalty Bearing Product. For clarity, Fully Burdened Manufacturing Cost excludes costs of excess capacity. Fully Burdened Manufacturing Cost shall be calculated in a manner consistent with Accounting Standards.

1.43 "Gene Therapy Construct". Gene Therapy Construct means any nucleic acid sequence that encodes an RNA or an amino acid sequence that is intended to be delivered to a targeted tissue to treat, prevent or ameliorate a disease or condition.

1.44 "GLP Tox Compound". GLP Tox Compound means a Research Compound that uniQure, in its sole discretion, elects to progress to GLP Tox Studies to be conducted by or on behalf of uniQure in accordance with Section 3.3(a).

1.45 "GLP Tox Study". GLP Tox Study means a formal toxicology study of a Research Compound conducted under Good Laboratory Practices that is required to obtain approval from a regulatory authority, whether the FDA or otherwise, to begin conducting Clinical Trials.

1.46 "Good Laboratory Practices". Good Laboratory Practices means the then-current good laboratory practice standards promulgated or endorsed by the FDA, as defined in 21

C.F.R. Part 58 (or such other comparable regulatory standards in jurisdictions outside the U.S. to the extent applicable to the relevant study, as they may be updated from time to time).

1.47 "Governmental Authority". Governmental Authority means any United States federal, state or local or any foreign government, or political subdivision thereof, or any multinational organization or authority or any authority, agency or commission entitled to exercise any administrative, executive, judicial, legislative, police, regulatory or taxing authority or power, any court or tribunal (or any department, bureau or division thereof), or any governmental arbitrator or arbitral body.

1.48 "Grant Letter". Grant Letter means each of the Option Agreements, dated as of even date herewith, by and between uniQure's Affiliate, uniQure B.V., and (a) in the first case, Dr. David Schaffer and (b) in the second case, Dr. David Kirn.

1.49 "IGT". IGT means Integrative Gene Therapeutics, Inc., a California corporation, which jointly owns with UC certain of the UC Patent Rights.

1.50 "Indication". Indication means any disease, condition or syndrome.

1.51 "Initial Research Term". Initial Research Term means the period commencing on the Effective Date and ending on the [**] anniversary thereof.

1.52 "Initiation". Initiation means, with respect to a Clinical Trial, the first dosing of a participant in such Clinical Trial.

1.53 "Invention". Invention means any new and useful process, article of manufacture, compound, composition of matter, formulation or apparatus, or any improvement thereof, discovery or finding, which is patentable.

1.54 "Invoice". Invoice means an original invoice sent by 4DMT to uniQure with respect to any payment due hereunder substantially in the form attached hereto as Schedule 1.54.

1.55 "Know-How". Know-How means (a) any scientific or technical information, results and data of any type whatsoever, in any tangible or intangible form whatsoever, that is not in the public domain, including databases, practices, methods, techniques, specifications, formulations, formulae, protein sequences, nucleic acid sequences, AAV Capsid Variants, AAV Capsid Variant Libraries, Gene Therapy Constructs, Compounds, knowledge, know-how, trade secrets, skill, experience, test data including pharmacological, medicinal chemistry, biological, chemical, biochemical, toxicological and clinical test data, analytical and quality control data, stability data, studies and procedures, and manufacturing process and development information, results and data, and (b) any biological, chemical, or physical material or composition of matter that is not in the public domain or otherwise generally available to the public.

1.56 "Law". Law means all laws, statutes, rules, codes, regulations, orders, judgments or ordinances applicable to a Party, this Agreement or the activities contemplated hereunder.

1.57 "Lead Optimization". Lead Optimization means the discovery phase dedicated to the evaluation of new AAV Capsid Variants derived from an AAV Capsid Variant Library following a Research Selection Process to identify one or more Research Compounds that meet Delivery Success Criteria.

1.58 "Licensed IP". Licensed IP means the 4DMT Intellectual Property, Core uniQure Intellectual Property, and Joint Intellectual Property.

1.59 "Materials". Materials means any tangible chemical or biological research materials that are provided or otherwise made available by one Party to the other Party under the terms of Section 3.4 for use in performance of the Research Program; provided, however, that Materials will not include any AAV Capsid Variants or AAV Capsid Variant Libraries.

1.60 "NDA". NDA means a New Drug Application or Biologics License Application filed with the FDA or any other application required for the purpose of marketing or selling or commercially using a therapeutic or prophylactic product to be filed with a Regulatory Authority in a non-U.S. country or group of countries, including a Product License Application or Marketing Authorization Application ("MAA") in the European Union or Japan.

1.61 "Net Sales". Net Sales means, with respect to a Royalty Bearing Product, the gross amount of sales of such Royalty Bearing Product invoiced by uniQure or its Affiliates to Third Parties, less the following to the extent related to such Royalty Bearing Product and incurred by such uniQure or its Affiliates and invoiced to the Third Party:

(a) sales returns and allowances actually paid, granted or accrued, including trade, quantity and cash discounts and any other adjustments, including those granted on account of price adjustments or billing errors;

(b) rejected goods, damaged or defective goods, recalls, returns;

(c) rebates, chargeback rebates, compulsory rebates, reimbursements or similar payments granted or given to wholesalers or other distributors, buying groups or health care insurance carriers;

(d) non-collectable receivables;

(e) customs or excise duties, sales tax, consumption tax, value added tax, and other taxes (except income taxes); or

(f) charges for packing, freight, shipping and insurance.

Each of the foregoing deductions shall be determined as incurred in the ordinary course of business in type and amount consistent with good industry practice and in accordance with

Accounting Standards on a basis consistent with uniQure's audited consolidated financial statements. For clarity, sales by uniQure or its Affiliates of a Royalty Bearing Product to a Third Party Distributor of such Royalty Bearing Product in a given country shall be considered a sale to a Third Party customer. All such discounts, allowances, credits, rebates, and other deductions shall be fairly and equitably allocated to the Royalty Bearing Products and other products of uniQure and its Affiliates such that the Royalty Bearing Product does not bear a disproportionate portion of such deductions.

In the event any Royalty Bearing Product is sold for consideration other than cash, Net Sales for such sale shall be the average price of such Royalty Bearing Product sold for cash during the relevant period in the relevant country.

In the event that any discount, reduction, payment or rebate is offered for a Royalty Bearing Product where such Royalty Bearing Product is sold to a Third Party customer as part of a grouped set of products, the applicable discount, reduction, payment or rebate for such Royalty Bearing Product in such arrangement shall be based on the weighted average discount, reduction, payment or rebate of such grouped set of products.

Any Royalty Bearing Products used for promotional or advertising purposes (in reasonable and customary amounts) or used for Clinical Trials or other research purposes shall not be included in Net Sales. Donations for charity reasons or compassionate use shall also not be included in Net Sales.

1.62 "Party" and "Parties". Party means uniQure or 4DMT individually, and Parties means uniQure and 4DMT collectively.

1.63 "Patent Rights". Patent Rights means patents, patent applications or provisional patent applications, utility models and utility model applications, petty patents, innovation patents, patents of addition, divisionals, continuations, continuation-in-part applications, continued prosecution applications, requests for continued examinations, reissues, renewals, reexaminations and extensions and supplementary protection certificates granted in relation thereto, in any country of the world. For clarity, Patent Rights shall include any Patent Right that claims priority to or common priority with such Patent Rights.

1.64 "Phase I Study". A Phase I Study is a human clinical trial conducted in any country that meets the requirements of 21 CFR §312.21(a). By way of example and not limitation, a Phase I Study is usually performed as a single or multiple dose clinical study in healthy volunteers or patients to assess specific administration, distribution, metabolism, excretion (ADME), safety and tolerability, bioavailability/bioequivalence or exploratory efficacy (in the sense of demonstrating "proof-of-principle") of an investigational drug, and the emphasis in Phase I is usually on safety and tolerability and it is typically used to plan patient dosing in Phase II clinical studies. For clarity, a Phase I Study may also represent the initial phase of a combined Phase Ib/II clinical study.

1.65 "Phase III Study". A Phase III Study is a human clinical trial conducted in any country that meets the requirements of 21 CFR §312.21(c). By way of example and not limitation, a Phase III Study is a large scale clinical study (usually several hundreds of patients)

performed after preliminary evidence suggesting effectiveness of the drug has been obtained in Phase II clinical studies, and it is intended to gather the pivotal information about effectiveness and safety that is needed to evaluate the overall benefit-risk relationship of the drug and, along with earlier Clinical Trials, to provide an adequate basis for Regulatory Approval. For clarity, a Phase III Study may also represent the second part of a combined Phase II/III clinical study.

1.66 "Pivotal Study". A Pivotal Study is a human clinical trial conducted in any country, the principal purpose of which is to establish safety and efficacy of a Royalty Bearing Product in patients with the applicable Indication and to gather the pivotal information about such safety and effectiveness that is needed to evaluate the overall benefit-risk relationship of the drug and, along with earlier Clinical Trials, to provide an adequate basis for Regulatory Approval. A Pivotal Study includes any human clinical trial intended as a pivotal study of such Royalty Bearing Product regarding such Indication, such as a phase II/III or phase IIb clinical trial, whether or not such study is a traditional Phase III Study.

1.67 "Pre-agreed". Pre-agreed means on terms that are determined by the JRSC in accordance with Section 2.5.

1.68 "Prior Confidentiality Agreement". Prior Confidentiality Agreement means the Two Way Confidentiality Disclosure Agreement between uniQure and 4DMT, dated August 26, 2013.

1.69 "Product". Product means any preparation in final form, either for sale by prescription, over-the-counter or any other method, or for administration to human patients in Clinical Trials, for any and all uses, and in any and all formulations and combinations, which preparation contains a Compound.

1.70 "Project Team". Project Team means the 4DMT and uniQure personnel involved in the Research Program, including the Project Leaders.

1.71 "Prosecution and Maintenance". Prosecution and Maintenance means, with respect to a Patent Right, the preparation, filing, prosecution and maintenance of such Patent Right, as well as reexaminations, reissues and the like with respect to such Patent Right, together with the conduct of interferences, the defense of oppositions and other similar proceedings with respect to the particular Patent Right; and "Prosecute and Maintain" shall have the correlative meaning.

1.72 "Regulatory Approval". Regulatory Approval means the technical, medical and scientific licenses, registrations, authorizations and approvals (including approvals of NDAs and labeling approvals) of any Regulatory Authority necessary for the distribution, marketing, promotion, offer for sale, use, import, export or sale of a Royalty Bearing Product in a regulatory jurisdiction.

1.73 "Regulatory Authority". Regulatory Authority means any applicable Governmental Authority involved in granting approvals for the manufacturing, marketing, reimbursement or pricing of a Royalty Bearing Product in the Territory or any portion thereof,

including the FDA and EMA (as applicable), and any successor Governmental Authority having substantially the same function.

1.74 "Research Compound". Research Compound means a Compound containing a Designated Capsid Variant that is the subject of activities under the Research Program.

1.75 "Research Plan". Research Plan means the research plan developed by the Parties that sets forth the activities to be undertaken during the Research Term with respect to the Research Program and the budget for such activities. The initial outline of the Research Plan is attached as Schedule 1.75.

1.76 "Research Program". Research Program means a program of collaborative research to be undertaken by the Parties pursuant to the Research Plan to identify optimized AAV Capsid Variants for use in the Field that demonstrate improved expression of the delivered Gene Therapy Construct in the targeted tissue as compared to currently available AAV Capsid Variants.

1.77 "Research Selection Process". Research Selection Process means the iterative evolution or isolation of lead AAV Capsid Variants from one or more 4DMT AAV Capsid Variant Libraries in cells (cultured or primary) *in vitro* or in animals *in vivo* intended to result in the identification of AAV Capsid Variants demonstrating Pre-agreed properties suitable to proceed into Lead Optimization using a Pre-agreed evaluation methodology and that are targeted to a specified target tissue. A given Research Selection Process is different from another Research Selection Process if such Research Selection Process was conducted to identify AAV Capsid Variants that specifically target a different tissue or are delivered by means of a different mode of administration (*e.g.*, such process was conducted to identify AAV Capsid Variants useful for intravenous, direct injection, subcutaneous or intrathecal delivery means).

1.78 "Research Term". Research Term means the Initial Research Term and, if applicable, the Extended Research Term.

1.79 "Research Year". Research Year means a twelve (12) month period beginning on the Effective Date or on any anniversary thereof.

1.80 "Royalty Bearing Compound". Royalty Bearing Compound means a Compound containing a Selected Capsid Variant.

1.81 "Royalty Bearing Product". Royalty Bearing Product means a Product containing a Royalty Bearing Compound.

1.82 "Royalty Term". Royalty Term means, with respect to a Royalty Bearing Product, on a Royalty Bearing Product-by-Royalty Bearing Product and a country-by-country basis, the period beginning on the First Commercial Sale of such Royalty Bearing Product in such country by uniQure or any of its Affiliates or Sublicensees, and ending on latest of: (a) the expiration of the last Valid Claim within the Licensed IP Covering such Royalty Bearing Product in such country, (b) the expiration of any applicable exclusivity, including orphan drug status or

data exclusivity, and any extension thereto, granted by a Regulatory Authority in such country with respect to such Royalty Bearing Product, or (c) the tenth (10^{th}) anniversary of the date of the First Commercial Sale by uniQure or any of its Affiliates or Sublicensees of such Royalty Bearing Product in such country.

1.83 "Selected Capsid Variant". Selected Capsid Variant means [**].

1.84 "Selection Process". Selection Process means the iterative evolution or isolation of lead AAV Capsid Variants from one or more AAV Capsid Variant Libraries in cells (cultured or primary) *in vitro* or in animals *in vivo* intended to result in the identification of AAV Capsid Variants demonstrating properties suitable to a specified target tissue. A given Selection Process is different from another Selection Process if such Selection Process was conducted to identify AAV Capsid Variants that specifically target a different tissue or are delivered by means of a different mode of administration (*e.g.*, such process was conducted to identify AAV Capsid Variants useful for intravenous, direct injection, subcutaneous or intrathecal delivery means).

1.85 "Sublicensee". Sublicensee means, with respect to uniQure, a Third Party to whom uniQure (or its Affiliate or another of its Sublicensees) has granted a license or sublicense under the Licensed IP to Develop, make and have made, use or Commercialize a Royalty Bearing Product; provided, however, that a Sublicensee shall not include any Third Party Distributor.

1.86 "Territory". Territory means all countries and territories in the world.

1.87 "Third Party". Third Party means an entity other than uniQure, 4DMT and their respective Affiliates.

1.88 "Third Party Distributor". Third Party Distributor means any Third Party that provides (but does not Develop) Royalty Bearing Products directly to customers under agreement with uniQure, its Affiliates or Sublicensees.

1.89 "UC AAV Capsid Variant". UC AAV Capsid Variant means any AAV Capsid Variant provided to 4DMT pursuant to the UCB Agreements.

1.90 "UC Patent Right". UC Patent Right means any Patent Right licensed to 4DMT pursuant to the UCB Agreements.

1.91 "UC Product". UC Product means a Royalty Bearing Product that is Covered by a UC Patent Right.

1.92 "UCB Agreements". UCB Agreements means (a) the Exclusive License and Bailment Agreement between 4DMT and the Regents of the University of California ("UC"), Agreement Control No. 2014-03-0089, dated December 19, 2013; (b) the Exclusive License and Bailment Agreement between 4DMT and UC, Agreement Control No. 2014-03-0090, dated December 19, 2013; and (c) the Agreement for Use of Certain Biological Materials between 4DMT and UC, Agreement Control No. 2014-30-0088, dated December 19, 2013, in each case in the form provided to uniQure by 4DMT as of the Effective Date.

1.93 "uniQure Intellectual Property". uniQure Intellectual Property means uniQure Know-How and uniQure Patent Rights.

1.94 "uniQure Know-How". uniQure Know-How means Know-How that is (a) Controlled by uniQure or its Affiliates as of the Effective Date or during the Term, and (b) necessary or useful to conduct the Research Program or to research, Develop, make and have made, use or Commercialize the relevant Selected Capsid Variant, Royalty Bearing Compound or Royalty Bearing Product. uniQure Know-How includes Core uniQure Know-How but does not include Joint Know-How.

1.95 "uniQure Patent Right". uniQure Patent Right means any Patent Right Controlled by uniQure or its Affiliates as of the Effective Date or during the Term that Covers uniQure Know-How. uniQure Patent Rights include Core uniQure Patent Rights but do not include Joint Patent Rights.

1.96 "Valid Claim". Valid Claim means (a) a claim of an issued patent that has not expired or been abandoned, or been revoked, held invalid or unenforceable by a patent office, court or other governmental agency of competent jurisdiction in a final and non-appealable judgment (or judgment from which no appeal was taken within the allowable time period), or (b) a claim within a patent application which application has not been pending for more than [**] years from the date of its priority filing date and which claim has not been irretrievably revoked, irretrievably cancelled, irretrievably withdrawn, held invalid or abandoned by a patent office, court or other governmental agency of competent jurisdiction in a final and non-appealable judgment (or judgment from which no appeal was taken within the allowable time period), or finally determined to be unallowable in a decision from which an appeal cannot or can no longer be taken; provided, however, that with respect to the UC Patent Rights licensed under the Exclusive License and Bailment Agreement between 4DMT and UC, Agreement Control No. 2014-03-0089, the foregoing [**] year limitation shall be extended to [**] years.

1.97 Additional Definitions. Each of the following definitions is set forth in the section of this Agreement indicated below:

Definition:	Section:
4DMT	Preamble
4DMT Indemnitees	9.5
Acquiring/Acquired Party	5.6(b)
Additional Cure Period	10.2(a)
Agreement	Preamble
Audited Party	6.7
Auditing Party	6.7
Bankruptcy Code	5.5
CNS	1.37
CREATE Act	7.10
Damages	9.5
Defaulting Party	10.2(a)

Definition:	Section:
Designated Capsid Variant	3.4(a)
Dispute	11.1
Effective Date	Preamble
Equipment Payment	6.2(c)
Excluded Claim	11.2
Executives	2.5(b)
Extended Research Term	3.1(c)
Failure to Amend	4.4(d)
Fair Market Value	6.5(b)(iii)
GAAP	1.9
GLP Tox Candidate Review Period	3.3(a)
IFRS	1.9
Initiating Party	7.6(d)
Joint Counsel	7.5
Joint Intellectual Property	7.2(a)
Joint Know-How	7.2(a)
Joint Patent Rights	7.2(a)
JRSC	2.2(a)
M&A Event	12.7
MAA	1.60
Non-Defaulting Party	10.2(a)
Orange Book	7.9(a)
Paragraph IV Certification	7.9(b)
Paragraph IV Proceeding	7.9(b)(ii)
Project Leader	2.1
Records	3.7(a)(i)
Replacement Product	6.3(b)(3)

SEC Filing	8.5(c)
Sublicense Consideration	6.5(b)
Sublicense Income Sharing Percentages	6.5(a)
Term	10.1
Third Party Claim	9.5
Third Party Competitive Product	4.4(a)
Third Party Proposal	4.4(a)
Third Party Proposed Products	4.4(a)
Third Party Proposer	4.4(a)
Trade Secret Election	7.3(b)
USPTO	7.10
UC	1.88
uniQure	Preamble
uniQure Indemnitees	9.6

ARTICLE II

GOVERNANCE

2.1 Project Leaders. Within [**] Business Days after the Effective Date, each Party will appoint (and provide written notice to the other Party of the identity of) a senior representative having a general understanding of pharmaceutical discovery and development issues to act as its project leader under this Agreement (each, a "Project Leader"). The Project Leaders will serve as the contact point between the Parties with respect to the Research Program, and will be primarily responsible for: (a) facilitating the flow of information and otherwise promoting communication, coordination of the day-to-day work and collaboration between the Parties; (b) providing single point communication for seeking consensus internally within the respective Party's organization; and (c) raising cross-Party or cross-functional disputes in a timely manner. The Project Leaders shall conduct regular telephone conferences as deemed necessary or appropriate, to exchange informal information regarding the progress of the Research Program. Each Party may change its designated Project Leaders from time to time upon prior written notice to the other Party. Each Project Leader may designate a substitute to temporarily perform the functions of that Project Leader by prior written notice to the other Party.

2.2 Joint Research Steering Committee.

(a) Composition. Promptly after the Effective Date, the Parties shall establish a joint research steering committee (the "JRSC"). The JRSC shall be comprised of at least [**] named representatives of uniQure and at least [**] named representatives of 4DMT, one of whom shall be David Schaffer (unless due to his death, illness or disability), or such other numbers as the Parties may agree in writing. As soon as practicable after the Effective Date (but in no event more than [**] Business Days after the Effective Date), each Party shall designate by written notice to the other Party its initial representatives on the JRSC. Each Party may replace one or more of its non-mandatory representatives, in its sole discretion, effective upon written notice to the other Party of such change. These representatives shall have appropriate technical credentials, experience and knowledge, and ongoing familiarity with the Research Program. The JRSC shall be disbanded upon expiration of the Research Term.

(b) Function and Powers of the JRSC. During the Research Term, the JRSC's responsibilities shall include: (i) approving the initial Research Plan and any amendment thereto, including allocation of tasks and resources; (ii) developing and approving the Candidate Success Criteria; (iii) developing and approving parameters for Animal POC; (iv) developing and approving parameters for Clinical POC; (v) determining the frequency of meetings of the Project Team, or subgroups of the Project Team, and the members of the Project Team to attend such meetings, which meetings are expected to occur at least [**], with such meetings expected to occur in person at least [**]; (vi) reviewing, approving procedures, and making recommendations regarding Lead Optimization; (vii) determining whether a Research Compound achieves the relevant Delivery Success Criteria; (viii) proposing Research Compounds that have achieved the Delivery Success Criteria for uniQure's acceptance as GLP Tox Compounds; (ix) providing a forum for discussion of the Research Plan, the status of the

Research Program, and relevant data; (x) serving as a forum for informal resolution of disagreements that may arise in the relation to the Parties' activities under the Research Program, including any disagreement within any subcommittee; (xi) determining and approving the overall strategy for publications and presentations pursuant to Section 8.4; and (xii) considering and acting upon such other matters as may be specified in this Agreement. Any decision made by the JRSC under this Section 2.2(b) shall be deemed a decision of the JRSC, as applicable, for purposes of this Agreement.

2.3 Subcommittees. The JRSC may establish and disband such subcommittees as deemed necessary by the JRSC. Each such subcommittee shall consist of the same number of representatives designated by each Party, which number shall be mutually agreed by the Parties. Each Party shall be free to change its representatives on written notice to the other Party or to send a substitute representative to any subcommittee meeting. Each Party's representatives and any substitute for a representative shall be bound by a written agreement with confidentiality obligations substantially the same as those set forth in ARTICLE VIII. The rules for the conduct of each subcommittee, and the scope of its responsibilities, shall be determined by the JRSC, provided that no subcommittee shall have the authority to bind the Parties hereunder, and each subcommittee shall report to the JRSC.

2.4 Meetings. The JRSC shall each hold at least [**]. Upon necessity, either Party shall be entitled to request additional meetings of the JRSC. Meetings of the JRSC shall be effective only if at least [**] representatives of each Party are present or participating. The location of meetings shall be as agreed by the Parties, and may be held in person, alternating locations between the Parties, or by telephone conference call or by videoconference; provided, however, that at least [**] meetings of the JRSC each Calendar Year are held in person. 4DMT's costs and expenses incurred in connection with preparing for and participating in all such meetings shall be paid for by uniQure in accordance with the budget for the Research Plan. Either Party may, from time to time, invite additional representatives or consultants to attend JRSC meetings; provided that at least [**] Business Days' prior written notice is given of a Party's intention to invite such other representatives or consultants and providing full details about the name, employer and professional background of such other representatives or consultants. Each representative and consultant participating in or attending a JRSC meeting shall be bound by a written agreement with confidentiality obligations substantially the same as those set forth in ARTICLE VIII. The JRSC shall be co-chaired by a representative from each Party. The chairpersons shall set the agendas for the JRSC meeting in advance. Within ten (10) Business Days prior to each scheduled meeting, each Party shall, in accordance with

Section 3.7(b), provide a report to the JRSC detailing its progress with respect to the Research Program. The Parties will rotate the responsibility for recording, preparing and issuing minutes for each JRSC meeting, to be circulated within [**] Business Days after each meeting.

2.5 Decision-making.

(a) Initial Dispute Resolution Procedures. Subject to the provisions of this Section 2.5, actions to be taken by the JRSC shall be taken only following a unanimous vote, with each Party, through its representatives, having one (1) vote. If any subcommittee fails to

reach unanimous agreement (with each Party, through its representatives, having one (1) vote) for a period in excess of [**] Business Days, the matter shall be referred to the JRSC.

(b) Referral of Unresolved Matters to Executives. If, in accordance with Section 2.5(a), the JRSC does not resolve any matter considered by it within [**]Business Days after the matter is first considered by it, the matter may be referred by either Party to the CEO of 4DMT and CEO of uniQure (the "Executives") to be resolved by negotiation in good faith as soon as practicable, but in no event later than [**] Business Days after referral. Such resolution, if any, of a referred issue by the Executives shall be final and binding on the Parties. Any decision made by the Executives under this Section 2.5(b) shall be deemed a decision of the JRSC for purposes of this Agreement.

(c) Final Decision-Making. If a dispute referred to the Executives pursuant to Section 2.5(b) has not been resolved in accordance with Section 2.5(b), then, subject to Section 2.5(d), uniQure shall have the final decision-making authority. Any decision made by uniQure pursuant to this Section 2.5(c) shall be deemed a decision of the JRSC for purposes of this Agreement.

(d) Exceptions. Notwithstanding Section 2.5(c), uniQure shall not have the right to exercise such decision-making authority (i) in a manner that excuses uniQure from any of its obligations specifically enumerated under this Agreement; (ii) in a manner that negates any consent rights or other rights specifically allocated to 4DMT under this Agreement; (iii) to resolve any dispute regarding whether a milestone event set forth in Section 6.3 has been achieved by uniQure by determining that such event has not been achieved; (iv) in a manner that would require 4DMT to perform activities (A) for which uniQure will not reimburse 4DMT's costs (except as expressly set forth in this Agreement), (B) that 4DMT has not agreed to perform as set forth in this Agreement or the Research Plan, or as otherwise agreed in writing by 4DMT, or (C) that require 4DMT to use any Know-How or other technology not contemplated in the Research Plan and that is not developed internally by 4DMT and with respect to the use of which 4DMT would owe a royalty or other payment; (v) in a manner that would change the total number of 4DMT FTEs or the allocation among the various technical disciplines as set forth in the Research Plan; (vi) in a manner that would reduce payments committed to 4DMT pursuant to this Agreement or take away 4DMT's right to perform activities that 4DMT has previously agreed to perform as set forth in the Research Plan; (vii) in a manner that would require 4DMT to perform any act that it reasonably believes to be inconsistent with any Law or any approval, order, policy, guidelines of a Regulatory Authority or ethical requirements or ethical guidelines; (viii) to determine that uniQure has fulfilled any obligation under this Agreement or that 4DMT has breached any obligation under this Agreement; or (ix) to amend the relevant Delivery Success Criteria. In the event that any matter set forth in the preceding clauses (i)-(ix) is unresolved through the JRSC and subsequently such dispute cannot be resolved by the Executives in accordance with Section 2.5(b), then either (A) for all such matters set forth in the preceding clauses (iv)-(vi), there shall be no change in the Research Plan or associated budget unless the Parties otherwise mutually agree in writing, (B) for all such matters set forth in the preceding clauses (i), (ii), (vii) and (viii), either Party may require the specific issue to be referred to binding arbitration pursuant to Section 11.2, or (C) for all such matters set forth in the preceding clauses (iii) and (ix), either Party may require the specific issue to be submitted to a

panel of external scientific experts to review the dispute pursuant to the remainder of this Section 2.5(d). Each Party shall select, upon either Party's request, one (1) external scientific expert within [**] Business Days after such request, and the two (2) so selected shall choose a third (3rd) external scientific expert within an additional [**] Business Days to resolve the dispute, and all three (3) shall serve as neutrals. Each expert must be free of any conflict of interest with respect to either or both Parties and their Affiliates and shall have expertise in the matters concerning the unresolved dispute. The decision of the external scientific expert panel shall be issued within [**] Business Days after nomination of the third external expert and shall be final and binding on the Parties. The Parties agree to share equally the cost of the proceedings, including fees of the panel members; provided, that each Party shall bear its own attorneys' fees and associated costs and expenses.

2.6 Limitations on JRSC Authority. The JRSC and any subcommittee shall have only the powers assigned expressly to it in this ARTICLE II and elsewhere in this Agreement, and shall not have any power to amend, modify or waive compliance with this Agreement. In furtherance thereof, each Party shall retain the rights, powers and discretion granted to it under this Agreement and no such rights, powers or discretion shall be delegated or vested in the JRSC or any subcommittee unless such delegation or vesting of rights is expressly provided for in this Agreement or the Parties expressly so agree in writing.

ARTICLE III

RESEARCH PROGRAM

3.1 General.

(a) Objectives. The objectives of the Research Program are to (i) identify and characterize AAV Capsid Variants and Research Compounds, (ii) optimize such AAV Capsid Variants and Research Compounds and (iii) conduct other research activities with respect to Research Compounds containing Gene Therapy Constructs of interest in place of marker or other proof-of-principle genes with which screening and AAV Capsid Variant optimization may have been performed, in each case to identify Research Compounds that meet the Delivery Success Criteria, with the objective of having such Research Compounds accepted by uniQure for Animal POC and subsequently as GLP Tox Compounds, consistent with the Candidate Success Criteria.

(b) Research Plan. The Parties shall agree to the Research Plan and shall conduct the Research Program in accordance with the Research Plan. The JRSC shall endeavor to approve the initial Research Plan (including its associated budget) within [**] days after the Effective Date, which initial Research Plan shall set forth the tasks to be undertaken by the Parties (including relevant technology to be used and Materials to be provided) under the Research Program.

(c) Extended Research Term. In the event that uniQure reasonably believes that the Parties will not complete the activities under the Research Plan during the Initial Research Term, then uniQure, at its sole discretion, may extend the Research Term to complete

the goals of such Research Plan as then in effect for an additional [**] month period from the expiration of the Initial Research Term (the "Extended Research Term"). uniQure may so extend the Research Term by giving written notice to 4DMT at least [**] months prior to the expiration of the Initial Research Term. The Parties shall mutually agree upon the number of FTEs at 4DMT needed to perform the research during the Extended Research Term, as well as out-of-pocket costs, and uniQure shall provide funding for such FTEs and out-of-pocket costs in accordance with Section 6.2(a) and, if the Parties are unable to agree on such matters prior to the expiration of the Initial Research Term, then the Research Term shall expire at the end of the Initial Research Term. The Parties may further extend the Extended Research Term by mutual written agreement.

3.2 Conduct of the Research Program.

(a) 4DMT and uniQure shall each use Commercially Reasonable Efforts to conduct the Research Program in accordance with the Research Plan. In addition, uniQure shall use Commercially Reasonable Efforts to assess reasonably promptly whether each Designated Capsid Variant provided to uniQure in connection with assessing the Delivery Success Criteria can be manufactured in insect cells.

(b) Either Party shall have the right to utilize the services of any Third Party to perform its obligations under the Research Plan to the extent that such Third Party is specifically approved in the Research Plan or otherwise approved by the JRSC, provided that any permitted Third Party must have entered into a written agreement with such Party that includes terms and conditions (i) protecting and limiting use and disclosure of Confidential Information at least to the same extent as under ARTICLE VIII, and (ii) requiring the Third Party and its personnel to assign to such Party all right, title and interest in and to any intellectual property (and intellectual property rights) created or conceived in connection with performance of subcontracted activities. Each Party shall remain at all times fully liable for its responsibilities under this Agreement.

(c) 4DMT and uniQure shall conduct the Research Program in accordance with all applicable Laws, including, if and as applicable, Good Laboratory Practices. Each Party hereby certifies that it will not employ or otherwise use in any capacity in performing any activity hereunder the services of any person or entity known to it to be debarred under 21 USC §335a.

(d) If the JRSC determines that it is desirable to transfer the AAV Capsid Variant Libraries into baculovirus, then prior to such transfer, the Parties will negotiate in good faith an amendment to this Agreement specifying the allocation of ownership of Materials, Know-How, and Patent Rights. Except as otherwise agreed by the Parties in writing, in no event shall 4DMT transfer the 4DMT AAV Capsid Variant Libraries to uniQure, and in no event shall uniQure transfer its baculovirus insect cell manufacturing Know-How to 4DMT.

3.3 Candidate Success Criteria.

(a) Within [**] days following the date on which the Research Plan is approved by the JRSC, the JRSC shall determine and approve the minimum Candidate Success

Criteria applicable to each class or series of Research Compounds. For clarity, the Candidate Success Criteria shall include the criteria necessary to move from testing AAV capsid gene-containing AAV Capsid Variants to marker or other Gene Therapy Construct of interest-containing AAV Capsid Variants, for testing for compatibility with or optimization or adaption to uniQure's insect cell manufacturing process, and for testing insect cell compatible Research Compounds for gene delivery efficiency in comparison to a relevant reference vector using Research Compounds and reference vector stocks prepared in insect cells by uniQure using uniQure SOPs and release specifications. The objectives of the Research Program will always be to identify the best possible AAV Capsid Variants for delivery of Gene Therapy Constructs to target cells, rather than to identify AAV Capsid Variants that merely meet the minimum Candidate Success Criteria specified in the Research Plan. Subsequently in the Research Program (*i.e.*, when AAV Capsid Variants have been accepted by uniQure as being ready for Animal POC testing or in parallel with the identification with lead AAV Capsid Variants for Lead Optimization), the JRSC will (i) agree on disease models for testing Gene Therapy Constructs of interest for efficacy against particular target diseases, (ii) agree on procedures for testing in these animal disease models and the Candidate Success Criteria in these models intending to result in data sufficient for submission to regulatory authorities, and (iii) recommend that Research Compounds meeting these criteria should proceed to GLP Tox Studies. The Candidate Success Criteria shall in all of cases (i)-(iii) be expected to be able to be met only using Research Compound stocks that have been prepared by uniQure in insect cells using standard uniQure SOPs in comparison to reference vectors also prepared by uniQure in the same way. Notwithstanding the foregoing, the Candidate Success Criteria shall be deemed to have been met for any Research Compound that uniQure advances into GLP Tox Studies.

(b) The JRSC may, from time to time during the Research Term, nominate a Research Compound that has achieved the Candidate Success Criteria for Animal POC (provided, however, that the JRSC may, as appropriate, nominate a Research Compound that has not achieved all the Candidate Success Criteria) for consideration as a GLP Tox Compound. uniQure will consider all data relating to the nominated Research Compound for designation as a GLP Tox Compound, including data generated by either uniQure or 4DMT pursuant to this Agreement. Such data shall include the results from all tests and other measures included in the Candidate Success Criteria and such other information and results as uniQure reasonably requests from 4DMT. Within [**] days after delivery to uniQure of such data (the applicable "GLP Tox Candidate Review Period"), uniQure shall provide 4DMT written notice whether uniQure accepts such nominated Research Compound as a GLP Tox Compound and intends to Develop and Commercialize such nominated Research Compound in accordance with the terms of this Agreement. Notwithstanding the foregoing, uniQure shall be deemed to have accepted as a GLP Tox Compound any Research Compound that it advances into pre-clinical Development conducted under Good Laboratory Practices.

(c) uniQure shall pay any applicable milestone payment as set forth in Section 6.3(a) for each Research Compound that uniQure advances into Animal POC as a potential GLP Tox Compound pursuant to this Section 3.3. For clarity, uniQure has the right to select any number of Research Compounds for Animal POC as potential GLP Tox Compounds without an additional payment for any such selection once the applicable milestone payments in

Section 6.3(a) have become payable for the first [**] Research Compounds for such Research Selection Process.

3.4 Selection of AAV Capsid Variants.

(a) Within [**] days after 4DMT provides uniQure with the list of AAV Capsid Variant sequences arising from each Research Selection Process and all other data arising from or relating to such Research Selection Process, uniQure shall submit by written notice to 4DMT a list specifying up to [**] AAV Capsid Variants from each such Research Selection Process (the "Designated Capsid Variants"). If uniQure has not provided such written notice to 4DMT within [**] days, 4DMT shall provide written notice to uniQure of the date that the foregoing [**] day period will expire, and the Parties will have the option to agree an extension by mutual consent, not to be unreasonably withheld.

(b) Prior to the [**] of the expiration of the Research Term, uniQure shall submit by written notice to 4DMT a list specifying up to [**] AAV Capsid Variants from the list of Designated Capsid Variants for each Research Selection Process. All AAV Capsid Variants included in such list shall be included as "Selected Capsid Variants," subject to the terms and conditions of this Agreement. For clarity, all modifications by uniQure to the Selected Capsid Variants and other modifications set forth in Section 1.83 shall also be deemed "Selected Capsid Variants" for purposes of the payment obligations under this Agreement. 4DMT shall provide written notice to uniQure if uniQure has not provided such list to 4DMT by the date that is [**] of the expiration of the Research Term.

(c) For clarity, the subset of Designated Capsid Variants not subsequently selected as Selected Capsid Variants may be used and licensed by 4DMT to Third Parties outside the Field, but only if they also arise from a Selection Process conducted outside the Field. Unless such subset of Designated Capsid Variants also arise from a Selection Process conducted outside the Field, 4DMT may not conduct any research using such subset of Designated Capsid Variants unless otherwise agreed under the Research Plan. For further clarity, Selected Capsid Variants may not be used, or licensed to Third Parties, by 4DMT or its Affiliates outside the Field.

3.5 Materials and Know-How Transfer/Use of Compounds.

(a) In order to facilitate the Research Program, each Party shall, as set forth in the Research Plan, provide to the other Party certain Materials and, subject to Section 3.6, Know-How Controlled by the supplying Party for use by the other Party in furtherance of the Research Program. In addition, 4DMT shall transfer to uniQure such quantities of Designated Capsid Variants as the JRSC may reasonably request from time to time during the Research Term to exercise its rights hereunder. All Materials and Know-How provided by one Party to the other Party remain the sole property of the supplying Party.

(b) All Materials transferred pursuant to the Research Program shall be used (i) only for the specific purpose provided for in the Research Plan, and (ii) solely under the control of the receiving Party. The Materials may not be used or delivered to or for the benefit of any Third Party without the prior written consent of the supplying Party, and shall not

be used in research or testing involving human subjects, except as expressly contemplated in the Research Plan or in accordance with this Agreement. All Materials shall be returned to the supplying Party or destroyed (at the election of the supplying Party) promptly after completion of the use permitted under this Agreement.

(c) THE MATERIALS ARE PROVIDED "AS IS" AND WITHOUT ANY REPRESENTATION OR WARRANTY, EXPRESS OR IMPLIED, INCLUDING ANY IMPLIED WARRANTY OF MERCHANTABILITY OR OF FITNESS FOR ANY PARTICULAR PURPOSE OR ANY WARRANTY THAT THE USE OF THE MATERIALS WILL NOT INFRINGE OR VIOLATE ANY PATENT OR OTHER PROPRIETARY RIGHT OF ANY THIRD PARTY.

(d) At the end of the Research Term, upon request by uniQure, 4DMT shall promptly provide to uniQure all quantities of the Royalty Bearing Compounds in 4DMT's possession and shall promptly destroy other Research Compounds.

3.6 Third Party Intellectual Property. The conduct of activities under the Research Plan will use Patent Rights or Know-How licensed by 4DMT pursuant to the UCB Agreements, subject to the terms and conditions of the UCB Agreements. 4DMT shall be solely responsible for all obligations under the UCB Agreements, including any and all payments and royalties due thereunder. In developing the Research Plan, the Parties shall discuss whether any Third Party Patent Rights or Know-How, other than Patent Rights or Know-How licensed by 4DMT pursuant to the UCB Agreements, will be utilized in the conduct of activities under the Research Plan. 4DMT shall disclose to uniQure the details of any restrictions on use or payment obligations of which it is aware that would be triggered by such use of Third Party Patent Rights or Know-How in the Research Program. If the Parties mutually agree to use any inventions claimed in any Patent Right or use any Know-How that is licensed to or has been acquired by 4DMT other than pursuant to the UCB Agreements, and if such use would require the payment of additional consideration to the Third Party from which the Patent Rights or Know-How was licensed or acquired, then such Patent Right or Know-How shall be deemed under the Control of 4DMT, provided that uniQure expressly agrees in writing to bear any such additional consideration actually to be paid by 4DMT to the Third Party (which amounts uniQure may offset pursuant to Section 6.4(c)(ii)) with respect to the Development, manufacture or Commercialization of Royalty Bearing Compounds or Royalty Bearing Products. For clarity, nothing in this Section 3.6 shall limit uniQure's rights to obtain from a Third Party, independent of 4DMT, a license or other right with respect to such Third Party's Patent Rights or Know-How.

3.7 Records and Reports.

(a) Records.

(i) 4DMT shall maintain records, in sufficient detail and in good scientific manner appropriate for patent and regulatory purposes, which shall fully and properly reflect all work done and results achieved in the performance of the Research Program by or on behalf of 4DMT (the "Records"), including the procedures, techniques and methodologies used, the progress made, and any Invention conceived or reduced to practice or

otherwise made within the scope of or in connection with the Research Program. As part of keeping the Records, 4DMT shall ensure that all of its personnel, and all of its agents that are involved in the Research Program, will keep accurate laboratory notebooks, which laboratory notebooks: (A) shall be duly signed, dated and witnessed; and (B) shall be created and maintained in accordance with its standard operating procedures that would be sufficient to allow for said laboratory

notebooks to be used in any proceeding before the United States Patent and Trademark Office or United States courts, in order to establish the date of invention for any Invention in accordance with the United States patent laws. During the Term, 4DMT shall, upon written request by uniQure, which shall not be unreasonably made: (1) make all Records available for inspection and review by uniQure during normal business hours in a timely manner; and (2) provide copies of the Records or any part thereof to uniQure, as reasonably requested by uniQure.

(ii) After a Research Compound has been accepted by uniQure as a GLP Tox Compound, uniQure shall have the right to request that a copy of the relevant portions of the laboratory notebooks relating to all stages of the generation of such GLP Tox Compound be provided by 4DMT to uniQure. After such request by uniQure, 4DMT shall provide such copies of the laboratory notebooks promptly to uniQure, which shall be maintained by uniQure as 4DMT's Confidential Information.

(b) Reports to the JRSC. Between [**] and [**] Business Days prior to each scheduled JRSC meeting, the Parties shall provide to the JRSC a written report on the progress of the Research Program, summarizing the work performed under the Research Program and evaluating the work performed in relation to the goals of the Research Program. Each Party shall provide such other information required by the Research Program or reasonably requested by the other Party and reasonably available, relating to the progress of the goals or performance of the Research Program.

ARTICLE IV

DEVELOPMENT AND COMMERCIALIZATION OF PRODUCTS; DILIGENCE

4.1 Responsibility. uniQure shall have full responsibility, at its sole expense, for the worldwide research, Development, manufacturing and Commercialization of Compounds and Products in the Field, subject to the payment obligations and other relevant terms and conditions of this Agreement.

4.2 Diligence. uniQure shall use Commercially Reasonable Efforts (itself or through an Affiliate or Sublicensee) to Develop, manufacture and Commercialize Royalty Bearing Compounds and Royalty Bearing Products in the Field (and, to the extent applicable under Section 4.4(b), Third Party Proposed Products and Third Party Competitive Products).

4.3 Progress Reports. After the end of the Research Term and continuing until the First Commercial Sale of a Royalty Bearing Product in any country in the Territory, uniQure shall provide, within [**] days after each [**] of each Calendar Year, a written progress report to 4DMT that summarizes the activities undertaken and the status of uniQure's

Development efforts with respect to the Royalty Bearing Compounds and Royalty Bearing Products (and, to the extent applicable under Section 4.4(b), Third Party Proposed Products and Third Party Competitive Products) during the [**] months that ended on the immediately prior [**], as applicable.

4.4 Proposed Products in the Field.

(a) If, at any time after the Research Term, a Third Party makes a bona fide proposal to 4DMT for Developing and Commercializing a Product in the Field (a "Third Party Proposed Product") using 4DMT Know-How or Joint Know-How, or the making, using or selling of which in the absence of an appropriate license would infringe a Valid Claim under the 4DMT Patent Rights or Joint Patent Rights, then 4DMT promptly shall notify uniQure of the proposal of such Third Party ("Third Party Proposer") and shall provide uniQure with such information regarding such Third Party proposal, including a development plan and a plan to finance such activities ("Third Party Proposal") as uniQure may reasonably request to evaluate such Third Party Proposal and its potential conflict with the ongoing efforts and future plans of uniQure. At any time after the Research Term, 4DMT may make a bona fide proposal to uniQure for Developing and Commercializing a Product in the Field (a "4DMT Proposed Product"), including a development plan and a plan to finance such activities. Within [**] days after receipt of a notice from 4DMT of a Third Party Proposal or 4DMT Proposed Product, uniQure shall notify 4DMT whether uniQure is conducting or is interested in conducting research or Development of such Third Party Proposed Product, 4DMT Proposed Product, or a Product that uniQure believes in good faith is or would be competitive with such Third Party Proposed Product or 4DMT Proposed Product (a "Competitive Product").

(b) If uniQure notifies 4DMT that uniQure is conducting or is interested in conducting research or Development of such Third Party Proposed Product, 4DMT Proposed Product or Competitive Product, uniQure shall, within [**] months after such notice, deliver to 4DMT a plan (including projected timelines) for the research and Development thereof and, thereafter, shall use Commercially Reasonable Efforts to research, Develop, manufacture and Commercialize such Third Party Proposed Product, 4DMT Proposed Product or Competitive Product consistent with the requirements under Section 4.2 in accordance with such plan. Each progress report provided to 4DMT under Section 4.3 from and after the date of uniQure's notice under this Section 4.4(d) shall contain a summary of the activities undertaken and the status of uniQure's research and Development efforts with respect to such Third Party Proposed Product, 4DMT Proposed Product, or Competitive Product during the [**] months that ended on the immediately prior [**], as applicable.

(c) If uniQure notifies 4DMT that uniQure is not conducting and is not interested in conducting research or Development of such Third Party Proposed Product, 4DMT Proposed Product, or Competitive Product, the Parties shall meet to determine whether the grant of an appropriate license, in the case of a Third Party Proposer, or an amendment to this Agreement, in the case of 4DMT, is necessary or appropriate in light of the circumstances, including consideration by the Parties of each of their respective rights and obligations. If 4DMT determines after such meeting and due consideration that the grant of a license to such Third Party Proposer or amendment to this Agreement, as applicable, is necessary or appropriate,

uniQure shall have [**] months after the date of receipt of written notice of such determination (or such longer time as shall be agreed to by the Parties in writing) to negotiate and enter into, as applicable, either (i) a sublicense under any relevant 4DMT Patent Rights to provide such Third Party Proposer with sufficient rights under the 4DMT Patent Rights (and no other intellectual property rights of any kind or Controlled by any person or entity) to research, Develop, manufacture and Commercialize the Third Party Proposed Product in the Field on commercially reasonable terms to be agreed by uniQure and such Third Party Proposer or (ii) an amendment to this Agreement to provide 4DMT with sufficient rights under the 4DMT Patent Rights to research, Develop, manufacture and Commercialize the 4DMT Proposed Product in the Field on commercially reasonable terms to be agreed by uniQure and 4DMT. uniQure and such Third Party Proposer or 4DMT, as applicable, shall define and agree on the uniQure Know-How and uniQure Patent Rights necessary to Develop or Commercialize AAV Capsid Variants to be licensed in such sublicense or amendment, as applicable.

(d) In the case of a Third Party Proposer, if uniQure fails to enter into such a sublicense agreement within such [**]month period, uniQure shall promptly (but in any event within [**] days after the end of such period) provide 4DMT in writing an explanation for such failure along with the proposed terms offered by uniQure to such Third Party Proposer. If 4DMT determines in its good faith judgment based on reasonable inquiry that the terms offered by uniQure to such Third Party Proposer were not commercially reasonable, 4DMT shall notify uniQure of such determination and provide uniQure with an additional [**] days to enter into a sublicense with such Third Party Proposer. If uniQure fails to enter into an agreement with such Third Party Proposer within such additional [**] day period, or in the case of a 4DMT Proposed Product, if uniQure and 4DMT fail to enter into an amendment to this Agreement within the [**] month negotiation period (a "Failure to Amend"), then 4DMT shall be free to dispute pursuant to ARTICLE XI whether uniQure has complied with its obligations under this Section 4.4.

(e) If uniQure or its Affiliates or Sublicensees ceases Development and Commercialization of all Royalty Bearing Compounds and all Royalty Bearing Products for a continuous period of [**] despite using Commercially Reasonable Efforts as required under Section 4.2, (i) uniQure shall provide written notice of such decision to 4DMT, and (ii) if 4DMT thereafter exercises its rights under this Section 4.4 with respect to a 4DMT Proposed Product and a Failure to Amend occurs, then 4DMT may submit the unresolved terms of such amendment for determination by binding arbitration pursuant to ARTICLE XI. In any such arbitration, the arbitrators shall be instructed to establish the commercially reasonable terms of such amendment within [**] days after the third arbitrator is appointed.

4.5 Pharmacovigilance. Within [**] months after the Effective Date, the Parties shall enter into an agreement governing the exchange of adverse event safety data (including post-marketing spontaneous reports) received by a Party and its Affiliates, including such data received from, in the case of uniQure, its Sublicensees or, in the case of 4DMT, its licensees, relating to any AAV Capsid Variant provided to uniQure by 4DMT hereunder in order to monitor the safety of all Compounds and Products and to meet reporting requirements with any applicable Regulatory Authority. Such data sharing agreement shall not require the sharing of data that would disclose confidential know-how or trade secrets of a Party or its Affiliates, or in the case of uniQure, its Sublicensees or, in the case of 4DMT, its licensees, if such data may

be cross-referenced, such as through a Drug Master File, to satisfy the requirements of Law and any applicable Regulatory Authority.

4.6 Marking. Prior to the issuance in the United States of Patent Rights included in the UC Patent Rights, uniQure agrees to mark Royalty Bearing Product(s) Covered by any UC Patent Right (or their containers or labels) sold in the United States under the licenses granted in this Agreement with the words "Patent Pending," and following the issuance in the United States of one or more Patent Rights included in the UC Patent Rights, with the patent numbers of the UC Patent Right(s) Covering such Royalty Bearing Product. All Royalty Bearing Products Covered by any UC Patent Right sold in other countries will be marked in such manner as to conform with the patent Laws and practice of such countries.

ARTICLE V

GRANTS OF RIGHTS

5.1 Licenses to uniQure.

(a) Research License to uniQure. Subject to the terms and conditions of this Agreement, 4DMT hereby grants to uniQure, and uniQure hereby accepts, during the Research Term and any applicable GLP Tox Candidate Review Period in effect as of the end of the Research Term, an exclusive (but not as to 4DMT), worldwide, royalty-free, non-sublicenseable license under the 4DMT Intellectual Property and 4DMT's interest in the Joint Intellectual Property, solely to (i) conduct activities assigned to uniQure under the Research Plan, (ii) evaluate Research Compounds, or (iii) evaluate the data developed in the conduct of activities under the Research Plan during the Research Term.

(b) Development and Commercialization License to uniQure. Subject to the terms and conditions of this Agreement, 4DMT hereby grants to uniQure, and uniQure hereby accepts, an exclusive (even as to 4DMT), worldwide, milestone- and royalty-bearing license, including the right to grant sublicenses in accordance with Section 5.3, under the 4DMT Intellectual Property and 4DMT's interest in the Joint Intellectual Property, to research (subject to 4DMT's retained rights to conduct research under the Research Program), Develop, make and have made, use and Commercialize Selected Capsid Variants, Royalty Bearing Compounds, and Royalty Bearing Products in the Field.

(c) Recordation. Following the Effective Date or at any time during the Term, 4DMT at the request and expense of uniQure shall promptly register or record the licenses granted to uniQure under this Agreement with the appropriate patent offices in all applicable countries of the Territory; provided that such registration or recordation specifies the applicable limitations of such license, and provided further that such registration shall have no effect on the allocation of Prosecution and Maintenance rights and obligations set forth in ARTICLE VII. In the event any of the licenses granted to uniQure under this Agreement are terminated in accordance with the terms of this Agreement, uniQure shall promptly take such actions and execute such documents as are reasonably requested by 4DMT to cancel such

registration(s) or recordation(s) in the applicable countries with respect to the terminated license grants.

5.2 Licenses to 4DMT.

(a) Research License to 4DMT. Subject to the terms and conditions of this Agreement, uniQure hereby grants to 4DMT, and 4DMT hereby accepts, during the Research Term and any applicable GLP Tox Candidate Review Period in effect as of the end of the Research Term, a non-exclusive, worldwide, royalty-free, non-sublicenseable license under the uniQure Intellectual Property, solely to the extent necessary to conduct activities assigned to 4DMT under the Research Plan.

(b) Grant-Back License to 4DMT Outside the Field. uniQure hereby grants to 4DMT, and 4DMT hereby accepts, a non-exclusive, worldwide, royalty-free license, including the right to grant sublicenses through multiple tiers, under the Patent Rights and Know-How Controlled by uniQure that (i) arise from activities that are conducted under this Agreement in connection with Royalty Bearing Compounds and Royalty Bearing Products in the course of making modifications to Selected Capsid Variants and (ii) claim or cover compositions of matter or general methods of use of Selected Capsid Variants that are applicable outside the Field (for clarity, excluding Patent Rights and Know-How claiming or covering (A) insect cell manufacturing technology, including

technology or sequence modifications for adapting AAV Capsid Variants to insect cells or insect cell expression vectors and systems, or (B) compositions, methods of manufacture, or methods of use of Gene Therapy Constructs, but for further clarity, including such Patent Rights and Know-How claiming or covering compositions combining Gene Therapy Constructs in general and AAV Capsid Variants in general or general methods of making or using such combinations of Gene Therapy Constructs and AAV Capsid Variants), to research, Develop, make and have made, use and Commercialize 4DMT AAV Capsid Variants (excluding Selected Capsid Variants), and Products containing such 4DMT AAV Capsid Variants, in all cases outside the Field. For the avoidance of doubt, 4DMT's practice of the foregoing license shall be subject to its exclusivity obligations set forth in Section 5.6. If any Patent Rights or Know-how subject to the foregoing license are subject to agreements between uniQure and a Third Party that require payments to be made to the Third Party by reason of the practice of the rights granted to 4DMT under this Section 5.2(b), such Patent Rights and Know-How shall only be deemed Controlled by uniQure if 4DMT agrees in writing to pay to uniQure the portion of the amounts due to such Third Party that is reasonably attributable to the practice of such rights.

5.3 Sublicenses. uniQure shall have the right to grant sublicenses under the license granted to it under Section 5.1(b) to Affiliates of uniQure and Third Parties; provided that any sublicense granted to a Third Party under this Agreement shall be pursuant to a written agreement that subjects such Sublicensee to all relevant restrictions and limitations set forth in this Agreement. uniQure shall provide 4DMT with the name and address of each Sublicensee of its rights under this ARTICLE V, the date of the grant of the sublicense and a description of the rights granted promptly after the execution and delivery of the sublicense agreement. uniQure shall remain responsible for the performance of its Sublicensees, and shall ensure that each Sublicensee complies with the applicable terms and conditions of this Agreement.

5.4 Rights Retained by the Parties. Except as expressly set forth in this Agreement, neither Party shall acquire any license or other intellectual property interest, by implication or otherwise, in any Confidential Information of the other Party or under any Patent Right or Know-How in which such other Party or its Affiliates has rights. Without limiting the generality of the foregoing, any of 4DMT's rights to 4DMT Intellectual Property not specifically licensed to uniQure shall be retained by 4DMT, and any of uniQure's rights to uniQure Intellectual Property not specifically licensed to 4DMT shall be retained by uniQure.

5.5 Section 365(n) of the Bankruptcy Code. All rights and licenses granted under or pursuant to any section of this Agreement are and will otherwise be deemed to be for purposes of Section 365(n) of the United States Bankruptcy Code (Title 11, U.S. Code), as amended or any comparable Law outside the United States (the "Bankruptcy Code"), licenses of rights to "intellectual property" as defined in Section 101(35A) of the Bankruptcy Code. The Parties will retain and may fully exercise all of their respective rights and elections under the Bankruptcy Code. Each Party agrees that the other Party, as licensee of such rights under this Agreement, will retain and may fully exercise all of its rights and elections under the Bankruptcy Code or any other provisions of applicable Law outside the United States that provide similar protection for "intellectual property." The Parties further agree that, in the event of the commencement of a bankruptcy proceeding by or against a Party under the Bankruptcy Code or analogous provisions of applicable Law outside the United States, the other Party will be entitled to a complete duplicate of (or complete access to, as appropriate) the intellectual property licensed to such other Party and all embodiments of such intellectual property, to the extent necessary for such other Party to practice the licenses granted to it pursuant to this Agreement under such intellectual property, which, if not already in such other Party's possession, will be promptly delivered to it upon such other Party's written request thereof. Any agreement supplemental hereto will be deemed to be "agreements supplementary to" this Agreement for purposes of Section 365(n) of the Bankruptcy Code.

5.6 Exclusivity.

(a) Of 4DMT. During the Term, 4DMT agrees to work exclusively with uniQure to research, Develop, manufacture and Commercialize AAV Capsid Variants, Compounds and Products in the Field, and Selected Capsid Variants, Royalty Bearing Compounds and Royalty Bearing Products in any field. In furtherance of the foregoing, 4DMT agrees, subject to the exceptions set forth in Section 5.6(b), that during the Term, it and its Affiliates shall not disclose any 4DMT Know-How or Joint Know-How, or grant rights to any

Third Party, to research, Develop, manufacture or Commercialize (i) any AAV Capsid Variant, Compound or Product in the Field or (ii) any Selected Capsid Variant, Royalty Bearing Compound or Royalty Bearing Product inside or outside the Field, in each case outside the scope of this Agreement. For clarity, notwithstanding the foregoing, 4DMT and its Affiliates may themselves, and may enter into licensing agreements and grant rights to Third Parties under 4DMT Intellectual Property, to research, Develop, manufacture or Commercialize any AAV Capsid Variants, Compound or Product (other than a Selected Capsid Variant, Royalty Bearing Compound or Royalty Bearing Product) outside the Field, as long as the relevant AAV Capsid Variant arose from a Selection Process conducted outside the Field. The Parties acknowledge that if 4DMT or its Affiliates or licensees research, Develop, manufacture or Commercialize AAV Capsid Variants that are not subject of the Research Program and products containing such AAV Capsid Variants that are demonstrated to be specifically targeted to cells not included in the CNS or the liver by any route of administration, as long as the relevant AAV Capsid Variant arose from a Selection Process conducted outside the Field, that such activities shall not be deemed to violate the terms of this Section 5.6(a). For purposes of further clarity and without limiting the foregoing, if an AAV Capsid Variant (other than a Selected Capsid Variant) is generated from a Selection Process for a specified target tissue other than the CNS or the liver, including for treatment of neoplastic and eye disorders, then such AAV Capsid Variant shall be deemed to be specifically targeted to cells not included in the CNS or the liver; provided that such AAV Capsid Variant may not then be used for the purpose of targeting cells in the CNS or the liver.

(b) Exceptions. The prohibitions set forth in Section 5.6(a) do not apply where 4DMT's or its Affiliates' (the "Acquiring/Acquired Party") involvement in such activity results from its acquisition of or by a Third Party (by merger or otherwise), and such former Third Party was engaged in such activity prior to such acquisition or merger; provided that (i) the Acquiring/Acquired Party shall not provide any such former Third Party with rights or access to the 4DMT Intellectual Property for use in connection with activities prohibited by this Section 5.6 if undertaken by the Acquiring/Acquired Party, and (ii) in the case where the Acquiring/Acquired Party acquires a Third Party (by merger or otherwise), the Acquiring/Acquired Party does not expand the scope of, or increase the financial commitment to, such Third Party activities from what it was immediately prior to the acquisition.

(c) uniQure Independent Activities. The Parties acknowledge and agree that uniQure will conduct research, Development, manufacturing and Commercialization activities independently of this Agreement, inside and outside of the Field, including with respect to AAV Capsid Variants, AAV Capsid Variant Libraries, Gene Therapy Constructs, Compounds and Products, and no provision of this Agreement shall apply to any such activity.

5.7 UCB Agreement Pass-Through Provisions. uniQure acknowledges that 4DMT has provided it with a copy of the executed UCB Agreements, and agrees that this Agreement is subject in all respects to the terms and conditions of the UCB Agreements. Notwithstanding the generality of the foregoing:

(a) uniQure acknowledges that UC (and, to the extent applicable, IGT) may publish any and all technical data resulting from any research performed by UC (and, to the

extent applicable, IGT) relating to the inventions disclosed in the UC Patent Rights, and UC (and, to the extent applicable, IGT) expressly reserves the right to use such inventions, UC AAV Capsid Variants and related technology for its educational and research purposes, to disseminate the UC AAV Capsid Variants and other tangible materials associated with, or required to practice such inventions or the UC Patent Rights to researchers at nonprofit institutions for their educational and research purposes, and to permit other nonprofit institutions to use the UC AAV Capsid Variants to practice the UC Patent Rights for education and research purposes.

(b) uniQure shall keep 4DMT informed of its large/small entity status, as defined in 15 U.S.C. 632.

(c) uniQure acknowledges that certain of the inventions disclosed in the UC Patent Rights were funded in part by the U.S. Government, and agrees that in accordance with 35 U.S.C. 204, to the extent required by Law, any products covered by the UC Patent Rights and sold in the United States will be substantially manufactured in the United States.

(d) uniQure acknowledges that 4DMT's exclusive rights, privileges, and licenses under the UCB Agreements will expire on the date of the last-to-expire Valid Claim under the UC Patent Rights covered in each agreement, respectively, unless earlier terminated.

(e) For any sublicense under the UC Patent Rights that uniQure grants under Section 5.3, uniQure shall ensure that (i) such further sublicense is subject to a written sublicense agreement and is bound by all of the applicable terms, conditions, obligations, restrictions and other covenants of the UCB Agreements that protect or benefit UC's (and, if applicable, the U.S. Government's) rights and interests to the same extent that this Agreement does, and (ii) it or the Sublicensee shall, within [**] days after executing such sublicense agreement, furnish to 4DMT for delivery to UC, subject to any confidentiality provisions, all material terms of such sublicense pertaining to UC's interests, including the Sublicensee's name and address, and indemnification of UC as provided in this Agreement.

(f) The Parties acknowledge and agree that upon termination of the UCB Agreements for any reason, uniQure's sublicenses under the UC Patent Rights under this Agreement will remain in effect and will be assigned to UC, except that UC will not be bound to perform any duties or obligations set forth herein that extend beyond the duties and obligations of UC set forth in the UCB Agreements.

(g) uniQure acknowledges that nothing contained in this Agreement will be construed as conferring any right to use in advertising, publicity or other promotional activities any name, trademark, trade name, or other designation of UC (including any contraction, abbreviation, or simulation of any of the foregoing), and that unless required by Law, regulation, or rules of a securities exchange, or consented to in writing by UC, the use by uniQure of the name "The Regents of the University of California" or the name of any University of California campus in advertising, publicity or other promotional activities is expressly prohibited.

ARTICLE VI

PAYMENTS; ROYALTIES AND REPORTS

6.1 Initial License Payment. In consideration of the rights to 4DMT Intellectual Property granted herein, uniQure shall pay to 4DMT non-creditable and non-refundable sums of: (a) One Hundred Thousand Dollars ($100,000) within [**] Business Days after the later of (i) the Effective Date and (ii) receipt of an Invoice for such amount and a duly signed original of this Agreement and, thereafter, (b) [**] Dollars ($[**]) within [**] Business Days after the later of (i) the JRSC's approval of the initial Research Plan (including its associated budget) and (ii) receipt of an Invoice for such amount.

6.2 Research Program Funding.

(a) Out-of-Pocket Costs. Following approval of the Research Plan (including its associated budget), uniQure shall fund all out-of-pocket costs to be incurred by 4DMT as specifically contemplated in the Research Plan, in accordance with the agreed-upon budget for such costs set forth in the Research Plan or as otherwise agreed to by uniQure. On or before the first date of each Calendar Quarter during the Research Term, uniQure shall pay 4DMT for such out-of-pocket costs to be incurred by 4DMT during such Calendar Quarter. Within [**] days after the end of each Calendar Quarter during the Research Term, 4DMT shall provide uniQure with a statement identifying such out-of-pocket costs incurred by 4DMT and paid to Third Parties in connection with the Research Program during such Calendar Quarter, in reasonable detail and with appropriate supporting documentation. If the supporting documentation shows that uniQure has overpaid or underpaid the out-of-pocket costs for such Calendar Quarter, 4DMT will, together with the supporting documentation, (i) send uniQure a credit note for the amount overpaid, upon which uniQure may credit the amount overpaid against any other payment due by uniQure under this Agreement, or if no other payment is due under this Agreement, 4DMT shall within [**] days refund the amount overpaid to uniQure, or (ii) send uniQure an Invoice for the amount underpaid, which uniQure shall pay within [**] days after uniQure's receipt of such Invoice. For clarity, no out-of-pocket costs will be paid by uniQure unless covered by an agreed-upon budget for such expenses set forth in the Research Plan or as otherwise agreed to by uniQure.

(b) 4DMT Committed FTEs. It is the Parties' intent that the Research Program will support the number of 4DMT FTEs in the performance of the activities under the Research Plan during the Research Term, as specified in the Research Plan and approved by the JRSC. Following approval of the Research Plan (including its associated budget), on or before the first day of each Calendar Quarter during the Research Term, uniQure shall pay 4DMT the FTE Costs for FTEs in the then-current Research Plan for such Calendar Quarter; provided that such payment may be pro rated in the first and last Calendar Quarters of the Research Term. Within [**] days after the end of each Calendar Quarter during the Research Term, 4DMT shall provide supporting documentation for the purpose of verifying the calculation of the FTE charges paid by uniQure for such Calendar Quarter. If the supporting documentation shows that uniQure has overpaid or underpaid the FTE payments for such Calendar Quarter, 4DMT will, together with the supporting documentation, (i) send uniQure a credit note for the amount

overpaid, upon which uniQure may credit the amount overpaid against any FTE or other payment due by uniQure under this Agreement, or if no other payment is due under this Agreement, 4DMT shall within [**] days refund the amount overpaid to uniQure, or (ii) send uniQure an Invoice for the amount underpaid, which uniQure shall pay within [**] days after uniQure's receipt of such Invoice. For clarity, no FTE Costs will be paid by uniQure unless covered by an agreed-upon budget for such FTEs set forth in the Research Plan or as otherwise agreed to by uniQure.

(c) Equipment Payment Reimbursement. Any amount paid by uniQure pursuant to Section 6.2(a) for the purchase of equipment ("Equipment Payment") shall be subject to partial reimbursement by 4DMT in accordance with this Section 6.2(c). For each of the first [**] Third Party collaborations 4DMT enters into after the Effective Date, 4DMT shall reimburse uniQure for a *pro rata* portion of the Equipment Payment based on the following formula: [**]. For example, if 4DMT conducts [**] Research Selection Processes hereunder and [**] Selection Processes for the first such Third Party collaboration in which such equipment was actually used, 4DMT shall reimburse uniQure for [**] percent ([**]%) of the Equipment Payments. If 4DMT subsequently conducts another [**] Selection Processes for the second Third Party collaboration in which such equipment was actually used, 4DMT shall reimburse uniQure for a further [**] percent ([**]%) of Equipment Payments, since the [**] Research Selection Processes it conducted for uniQure represents [**] of the aggregate Selection Processes conducted by 4DMT for uniQure and for the first [**] Third Party collaborations 4DMT entered into after the Effective Date. 4DMT shall pay uniQure any such amount payable under this Section 6.2(c) within [**] days after the end of the Calendar Quarter during which 4DMT conducted any Selection Process for either of the first [**] Third Party collaborations 4DMT enters into after the Effective Date in which such equipment was actually used, and shall contemporaneously provide uniQure with a written report detailing the calculation of such amount.

6.3 Research and Development Milestone Payments.

(a) Research Milestone Payments. uniQure shall pay to 4DMT the non-refundable, non-creditable milestone payments set forth below upon the occurrence of the applicable milestone event on a Research Selection Process-by-Research Selection Process basis:

Milestone Event		Payment
(i)	[**]	[**]
(ii)	[**]	[**]
(iii)	[**]	[**]

For clarity, if the milestone event set forth in Section 6.3(a)(i) is achieved by a first Research Compound and the corresponding milestone payment is made to 4DMT, and the milestone is subsequently achieved by a second or a third Research Compound from the same Research Selection Process, then uniQure shall not be obligated to pay

the milestone payment set forth in Section 6.3(a)(i) upon such achievement and shall pay instead the milestone payment set forth in Section 6.3(a)(ii) for the second such Research Compound and the milestone payment set forth in Section 6.3(a)(iii) for the third such Research Compound. For clarity, no milestone payment under this Section 6.3(a) shall by payable for any achievement by a fourth or subsequent Research Compound from the same Research Selection Process.

(b) Development Milestone Payments. uniQure shall pay to 4DMT the non-refundable, non-creditable milestone payments set forth below upon the occurrence of the applicable milestone event, on a Royalty Bearing Product-by-Royalty Bearing Product and Indication-by-Indication basis:

Milestone Event		Payment
(i)	[**]	[**]
(ii)	[**]	[**]
(iii)	[**]	[**]
(iv)	[**]	[**]
(v)	[**]	[**]

(1) The achievement of milestone events set forth in Section 6.3(b)(i) through Section 6.3(b)(v) shall be determined on a Royalty Bearing Product-by-Royalty Bearing Product and Indication-by-Indication basis. The achievement by a Royalty Bearing Product of the same milestone event that had been achieved by a different Royalty Bearing Product shall trigger another payment of the applicable milestone payment. The achievement by a Royalty Bearing Product of the same milestone event with respect to a different Indication also shall trigger another payment of the applicable milestone payment. For purposes hereof, one Royalty Bearing Product shall be different from another Royalty Bearing Product if it contains a different delivered nucleic acid and is Developed to treat a different Indication. An Indication that is a subset of or encompasses within its scope another Indication shall not be deemed a different Indication from such other Indication.

(2) The achievement of any later-listed milestone event set forth in Section 6.3(b)(ii) through Section 6.3(b)(v) shall cause payment for any earlier-listed milestone event in Section 6.3(b)(i) through Section 6.3(b)(iv) to become payable if such earlier-listed milestone event had not already become payable.

(3) Notwithstanding the foregoing, if (A) Development of a Royalty Bearing Product is terminated after any milestone payment set forth in Section 6.3(b)(i) through Section 6.3(b)(v) has been

made with respect to such Royalty Bearing Product and (B) another Royalty Bearing Product is selected to replace the terminated Royalty Bearing Product for the same Indication ("Replacement Product"), then there shall be no payment due upon achievement of the same milestone by such Replacement Product for which 4DMT already received a milestone payment for the original Royalty Bearing Product. For purposes of this Section 6.3(b), if Development of a Royalty Bearing Product is terminated, any Royalty Bearing Product containing a Research Compound from the same Research Selection Process, and Developed for the same Indication as such terminated Royalty Bearing Product, shall be deemed a Replacement Product.

(c) Payment of Milestones. uniQure shall provide written notice to 4DMT of the achievement of any milestone event set forth in Section 6.3(a) or Section 6.3(b) within [**] Business Days after the occurrence of such milestone event, and shall make the corresponding milestone payment within [**] days after receipt of an Invoice therefor.

6.4 Royalties. On a Royalty Bearing Product-by-Royalty Bearing Product basis, uniQure shall pay to 4DMT royalties on worldwide Net Sales as provided in this Section 6.4:

(a) Royalty Rate. uniQure shall pay to 4DMT royalties on Net Sales of each Royalty Bearing Product by uniQure and its Affiliates equal to [**] percent ([**]%) of all such Net Sales of such Royalty Bearing Product achieved during the applicable Calendar Year.

(b) Royalty Term. uniQure's royalty obligations to 4DMT under this Section 6.4 shall be in effect on a country-by-country and Royalty Bearing Product-by-Royalty Bearing Product basis during the relevant Royalty Term. Upon expiration of the Royalty Term for a Royalty Bearing Product in a country, the license under Section 5.1(b) shall be fully paid-up, irrevocable, perpetual and exclusive under the relevant Licensed IP for such Royalty Bearing Product in such country.

(c) Royalty Adjustments.

(i) Non-Patented Product. If a Royalty Bearing Product is sold in a country and the composition of matter, formulation, or method of use of such Royalty Bearing Product is not Covered by a Valid Claim within the Licensed IP in such country at the time of sale, then the royalty rate for such Royalty Bearing Product in such country shall be reduced by [**] percent ([**]%) of the applicable rate determined pursuant to Section 6.4(a), unless such Royalty Bearing Product embodies an Invention with respect to which uniQure made a Trade Secret Election, in which case no such reduction shall apply.

(ii) Third Party Offset. If uniQure is required, in order to avoid infringement of any Patent Right not licensed hereunder that Covers the composition of matter, formulation, or method of use of a Royalty Bearing Product, to obtain a license from a Third Party in order to Develop, make, have made, use or Commercialize such Royalty Bearing Product in a country in the Territory and to pay a royalty or other consideration under such license (including in connection with the settlement of a patent infringement claim), then the royalty payments due under Section 6.4(a) with respect to Net Sales for such Royalty Bearing

Product in such country shall be reduced by [**] percent ([**]%) of the amounts payable by uniQure to such Third Party for such license that are reasonably and appropriately allocable to such Royalty Bearing Product in such country, provided that in no event shall the foregoing reduce the amount of royalties payable to 4DMT in any Calendar Quarter by more than [**]percent ([**]%) of the amount determined pursuant to Section 6.4(a), as adjusted by application of the terms of Section 6.4(c)(i).

(iii) Limits on Deductions. Except as expressly provided in this Section 6.4, there shall not be any offset to or deduction from the royalties payable pursuant to this Section 6.4. Notwithstanding Sections 6.4(c)(i) and (ii) to the contrary, in no event shall the cumulative effect of the deductions in Sections 6.4(c)(i) and (ii) reduce the royalties to less than [**] percent ([**]%) of the amounts determined pursuant to Section 6.4(a).

6.5 Sublicense Consideration.

(a) uniQure shall pay to 4DMT the following percentages ("Sublicense Income Sharing Percentages") of Sublicense Consideration received by uniQure for sublicenses under the Licensed IP under this Agreement:

(i) [**] percent ([**]%) for any sublicense that (A) is granted prior to initiating Animal POC for any Compound or Product that is subject of the sublicense and (B) does not require uniQure to manufacture any such Compound or Product for Clinical Trial or commercial purposes;

(ii) [**] percent ([**]%) for any sublicense that (A) is granted prior to initiating Animal POC for any Compound or Product that is subject of the sublicense and (B) requires uniQure to manufacture any such Compound or Product for Clinical Trial or commercial purposes;

(iii) [**] percent ([**]%) for any sublicense that does not meet the criteria set forth in Section 6.5(a)(i) or Section 6.5(a)(ii) above;

provided, however, that none of subsections (i), (ii) or (iii) shall result in uniQure paying to 4DMT under this Section 6.5 a percentage of any Sublicense Consideration consisting of royalties from Sublicensees on sales of UC Products during the applicable Royalty Term that is less than [**] percent ([**]%) of Net Sales by such Sublicensee of such UC Products.

(b) The term "Sublicense Consideration" shall mean consideration of any kind received by uniQure from a Sublicensee for the grant of a sublicense under this Agreement, such as upfront fees, royalties or milestone fees and including any premium paid by the Sublicensee over the Fair Market Value (as defined below) for stock of uniQure in consideration for such sublicense; provided, however, the following are not included in Sublicense Consideration:

(i) Support for activities of uniQure relating to the research, Development, manufacturing or Commercialization of Royalty Bearing Products, which shall not exceed the fully burdened cost (and in the case of manufacturing costs, the Fully Burdened

Manufacturing Cost) for undertaking such activities performed by or for uniQure (including Third Parties on uniQure's behalf) by more than [**] percent ([**]%);

(ii) Proceeds derived from debt financing and any loans to uniQure by the Sublicensee;

(iii) Consideration received for the purchase of stock in uniQure or its Affiliate to the extent that the price per share for such equity does not exceed the Fair Market Value of such stock. The term "Fair Market Value" shall mean the average price at which the stock in question is publicly trading at for [**] days prior to the earlier of (A) the date of the announcement of its purchase by the Sublicensee or (B) the date of its purchase by the Sublicensee, or if the stock is not publicly traded, the value of such stock as determined in good faith by the Board of Directors of uniQure or its applicable Affiliate as of the time of receipt of payment; and

(iv) Reimbursement of uniQure's patent costs related to Patent Rights.

6.6 Reports; Payments. Within [**] days after the end of each Calendar Quarter during which there are Net Sales giving rise to a payment obligation under Section 6.4 or uniQure received Sublicense Consideration giving rise to a payment obligation under Section 6.5, (a) uniQure shall submit to 4DMT a report (i) identifying for each Royalty Bearing Product the Net Sales for such Royalty Bearing Product for each country for such Calendar Quarter, the calculation of royalties (including gross sales and all deductions taken from gross sales and all reductions pursuant to Section 6.4(c)), and the royalties payable to 4DMT and (ii) identifying the Sublicense Consideration received by uniQure in such Calendar Quarter and the one or more Sublicense Income Sharing Percentages applicable to such Sublicense Consideration, and (b) uniQure shall pay to 4DMT all royalties payable by uniQure under Section 6.4 and portions of Sublicense Consideration payable by uniQure under Section 6.5.

6.7 Books and Records; Audit Rights. Each Party (the "Audited Party") shall keep (and shall cause its Affiliates and Sublicensees to keep) complete, true and accurate books and records in accordance with its Accounting Standards in sufficient detail for the other Party (the "Auditing Party") to determine the payments due and costs incurred under this Agreement. Each Auditing Party shall have the right, [**] at its own expense, to have an independent, certified public accounting firm of nationally recognized standing, selected by the Auditing Party and reasonably acceptable to the Audited Party, review any such records of the Audited Party in the location(s) where such records are maintained by the Audited Party upon reasonable notice (which shall be no less than [**] days prior notice) and during regular business hours and under obligations of strict confidence, for the sole purpose of verifying the accuracy of the amounts paid under this Agreement within a [**] Calendar Year period preceding the date of the request for review. The report of such accounting firm shall be limited to a certificate stating whether any report made or invoice or payment submitted by the Audited Party during such period is accurate or inaccurate, the actual amounts of 4DMT out-of-pocket expenses under Section 6.2(a), FTE Costs under Section 6.2(b), Equipment Payment reimbursements under Section 6.2(c), and any payments under Section 3.6, and the amount of any Net Sales, milestone, royalty

or other payment discrepancy. No other information shall be provided to the Auditing Party. The Audited Party shall receive a copy of each such report concurrently with receipt by the Auditing Party. Should such inspection lead to the discovery of a discrepancy to the Auditing Party's detriment, the Audited Party shall pay the amount of the discrepancy within [**] days after its receipt from the accounting firm of the certificate showing the amount of the discrepancy. The Auditing Party shall pay the full cost of the review unless (a) uniQure was the Audited Party and the audited determined an underpayment of milestones or royalties which is greater than [**] percent ([**]%) of the amount due for the applicable period, in which case uniQure shall pay the reasonable costs charged by such accounting firm for such review, or (b) 4DMT was the Audited Party and the audit determined an overpayment of 4DMT out-of-pocket expenses under Section 6.2(a) or FTE Costs under Section 6.2(b), or underpayment of Equipment Payment reimbursements under Section 6.2(c), which is greater than [**] percent ([**]%) of the amount due for the applicable period, in which case 4DMT shall pay the reasonable costs charged by such accounting firm for such review. Any overpayment of royalties by uniQure revealed by an inspection shall be fully creditable against future royalty payments under Section 6.4.

6.8 Withholding Taxes. Subject to the provisions of Section 12.7, if Laws require withholding by uniQure of taxes imposed upon 4DMT on account of any royalty or other payment paid under this Agreement, such taxes shall be deducted by uniQure as required by Law from such remittable royalty or other payment and shall be paid by uniQure to the proper tax authorities; provided that before making any such deduction or withholding, uniQure shall give 4DMT notice of the intention to make such deduction or withholding, which notice shall include the authority, basis and method of calculation for the proposed deduction or withholding, and shall be provided to the extent practicable at least a reasonable period of time before such deduction or withholding is required, in order for 4DMT to obtain reduction of or relief from such deduction or withholding. Official receipts of payment of withholding taxes shall be secured and sent to 4DMT as evidence of such payment. The Parties shall exercise their best efforts to ensure that any withholding tax imposed is reduced as far as possible under the provisions of any relevant tax treaty.

6.9 United States Dollars. All dollar ($) amounts specified in this Agreement are United States dollar amounts.

6.10 Payment Method and Currency Conversion. Except as otherwise provided herein, all payments due to a Party hereunder shall be due and payable within [**] days after receipt of an invoice from the other Party and shall be paid via a bank wire transfer to such bank account as such Party shall designate. For the purposes of determining the amount of any payment due to 4DMT hereunder for the relevant Calendar Quarter under Section 6.4 or Section 6.5, amounts received by uniQure in any foreign currency shall be converted into United States dollars in accordance with the normal business practice of uniQure, as applied consistently across its business.

6.11 Blocked Payments. If, by reason of applicable Laws in any country in the Territory, it becomes impossible or illegal for uniQure or any of its Affiliates or Sublicensees to transfer, or have transferred on its behalf, royalties or other payments to 4DMT, uniQure shall

promptly notify 4DMT of the conditions preventing such transfer and such royalties or other payments shall be deposited in local currency in the relevant country to the credit of 4DMT in a recognized banking institution with a good creditworthiness, such banking institution to be designated by 4DMT or, if none is designated by 4DMT within [**] days, in a recognized banking institution selected by uniQure or its Affiliate or Sublicensee, as the case may be, and identified in a written notice given to 4DMT. If so deposited in a foreign country, uniQure shall provide, or cause its Affiliate or Sublicensee to provide, reasonable cooperation to 4DMT so as to allow 4DMT to assume control over such deposit as promptly as practicable.

6.12 Late Payments. Any payment not made within [**] Business Days after the due date for such payment pursuant to the terms of this Agreement shall bear interest at a rate of the thirty-day U.S. dollar LIBOR rate effective for the date that payment was due (as published in The Wall Street Journal, Eastern Edition) plus [**] per annum. Calculation of interest will be made for the exact number of days the payment was past due based on a year of 360 days (actual days/360).

ARTICLE VII

PATENTS

7.1 Disclosure. Each Party shall promptly disclose to the other Party any Inventions that it or its Affiliates or Sublicensees or their employees, independent contractors, or agents solely or jointly make, conceive, reduce to practice, or otherwise discover under this Agreement, and each Party shall maintain and make available to the other Party records regarding any Inventions that it has an obligation to assign under Section 7.2(a).

7.2 Ownership.

(a) uniQure shall solely own all Core uniQure Intellectual Property, and 4DMT shall solely own all Core 4MDT Intellectual Property. Without additional consideration, each Party shall assign and hereby does assign to the other Party such of its right, title, and interest in and to such Patent Rights (and shall require its Affiliates and Sublicensees, and all employees, independent contractors and their employees, and agents of such Party and its Affiliates and Sublicensees to so assign to the other Party such of their right, title, and interest) as is necessary to effectuate the allocation of right, title, and interest as set forth in this Section 7.2(a).

(b) Except as set forth in Section 7.2(a), as between the Parties, (i) each Party shall solely own all Know-How and Inventions invented solely by employees, agents and consultants of such Party or its Affiliates, and any Patent Right related thereto, subject to the licenses granted under ARTICLE V, and (ii) Know-How and Inventions invented jointly by employees, agents, or consultants of the Parties or their Affiliates ("Joint Intellectual Property", which includes any Patent Right Covering such Know-How and Inventions ("Joint Patent Rights") and any Know-How included in such Joint Intellectual Property ("Joint Know-How")) shall be jointly owned, subject to the licenses granted under ARTICLE V. Inventorship shall be

determined in accordance with U.S. patent Laws for purposes of determining ownership in accordance with the foregoing.

(c) Except as expressly provided in this Agreement, and subject to any restriction herein (including the licenses and exclusivity granted under ARTICLE V), (i) each joint owner may engage in research, Development, manufacturing and Commercialization activities relating to Joint Intellectual Property, and (ii) each may assign, license, sell or otherwise encumber or transfer any such interest without the prior written approval of the other Party and without obligation to account or provide compensation to the other Party.

7.3 uniQure Prosecution and Maintenance of Patent Rights.

(a) uniQure shall be solely responsible for the Prosecution and Maintenance of the uniQure Patent Rights, including the Core uniQure Patent Rights, at its sole expense and its sole discretion. uniQure shall give 4DMT an opportunity to review the text of each application, office action response or other substantive document for [**] (but not any other uniQure Patent Right) before filing with any patent office in the Territory, shall consider 4DMT's reasonable comments with respect thereto, and shall supply 4DMT with a copy of each such application, office action response or other substantive document as filed, together with notice of its filing date and serial number.

(b) uniQure shall have the sole right to determine whether any patent application is filed with respect to any Core uniQure Know-How and whether to maintain any Invention included in the Core uniQure Know-How as a trade secret. uniQure shall provide 4DMT with written notice if uniQure elects not to file a patent application claiming any particular Invention included in the Core uniQure Know-How specifically relating to compositions of matter of, methods of use of, or methods of making any Selected Capsid Variant because uniQure prefers to maintain such Invention as a trade secret (each, a "Trade Secret Election").

(c) uniQure shall notify 4DMT at least [**] days in advance of any applicable deadline if (i) uniQure decides that it does not wish to continue the Prosecution and Maintenance of a [**] for which no substitute has been filed, or (ii) uniQure decides that it intends to abandon claim scope in a [**], which claim scope is intended to be maintained by 4DMT, in which case, with respect to this clause (ii), 4DMT may assume responsibility for such claim scope by filing a divisional application restricted to such claim scope. In such cases (i) or (ii), uniQure shall allow 4DMT to assume responsibility for Prosecution and Maintenance of such Core uniQure Patent Right or divisional application at 4DMT's expense. If 4DMT assumes such responsibility, then 4DMT may designate any counsel of its choice reasonably acceptable to uniQure to handle the Prosecution and Maintenance of such Core uniQure Patent Right or divisional application (which shall otherwise continue to be part of the Core uniQure Patent Rights).

7.4 4DMT Prosecution and Maintenance of Patent Rights. 4DMT shall be solely responsible for the Prosecution and Maintenance of the 4DMT Patent Rights, including the Core 4DMT Patent Rights, at its sole expense and its sole discretion. 4DMT will reasonably

inform uniQure regarding the Prosecution and Maintenance of 4DMT Patent Rights (including in any case, an update at least [**]). Notwithstanding the foregoing, the Parties acknowledge that UC will handle the Prosecution and Maintenance of the UC Patent Rights in accordance with the terms of the UCB Agreements.

7.5 Prosecution and Maintenance of Joint Patent Rights. The Prosecution and Maintenance of any Joint Patent Right shall be through a mutually selected patent counsel. Within [**] days following the Effective Date, the Parties shall agree on a patent counsel ("Joint Counsel") who shall be engaged by both Parties for the Prosecution and Maintenance of all such Joint Patent Rights. The following terms shall apply to each Joint Patent Right:

(a) The Parties shall instruct Joint Counsel to conduct its activities as follows: The Joint Counsel shall give uniQure and 4DMT (or each Party's designee) an opportunity to review the text of each application, office action response or other substantive document for a Joint Patent Right before filing with any patent office in the Territory, shall incorporate uniQure's and 4DMT's (or each Party's designee) reasonable comments with respect thereto, and shall supply uniQure and 4DMT (or each Party's designee) with a copy of each such application, office action response or other substantive document as filed, together with notice of its filing date and serial number. In the event that 4DMT and uniQure provide Joint Counsel with conflicting instructions regarding the Prosecution and Maintenance of a Joint Patent Right, Joint Counsel shall make the Parties aware of such conflicting instructions and, if the Parties are not able to resolve such conflict within a reasonable time prior to the applicable filing deadline, the Joint Counsel shall take such action as would reasonably be expected to maximize the scope, extent and coverage of such Joint Patent Right.

(b) Both Parties shall cooperate with Joint Counsel in Prosecution and Maintenance of patent applications for Joint Patent Rights, including providing Joint Counsel with data and other information as appropriate with respect thereto.

(c) Joint Counsel shall keep uniQure and 4DMT advised of the status of the Prosecution and Maintenance of Joint Patent Rights, including actual and prospective patent filings for Joint Patent Rights, and shall provide each Party with advance copies of any and all papers related thereto. Joint Counsel shall promptly give notice to uniQure and 4DMT of the grant, lapse, revocation, surrender, invalidation or abandonment of any Joint Patent Right.

(d) The Parties shall equally share all fees and costs charged by Joint Counsel with respect to the Prosecution and Maintenance of Joint Patent Rights and all other mutually agreed and approved out-of-pocket costs and expenses incurred by either Party in connection with such Prosecution and Maintenance of Joint Patent Rights.

(e) uniQure shall notify 4DMT and Joint Counsel at least [**] days in advance of the next deadline if (A) uniQure decides that it does not wish to continue paying for the Prosecution and Maintenance of a particular Joint Patent Right for which no substitute has been filed, or (B) uniQure decides that it intends to abandon claim scope in a Joint Patent Right which claim scope is intended to be maintained by 4DMT, in which case, with respect to this clause (B), 4DMT may assume responsibility for such claim scope by filing a divisional

application restricted to such claim scope. In such cases (A) or (B), uniQure shall allow 4DMT to assume responsibility for Prosecution and Maintenance of the respective Patent Rights, including payments incurred after [**] days after receipt of uniQure's notice. If 4DMT assumes such responsibility, then: (i) 4DMT may designate any counsel of its choice to handle the Prosecution and Maintenance of such Joint Patent Right or of the divisional application and it shall cease to be a part of the Joint Patent Rights; (ii) uniQure shall lose its licenses to such former Joint Patent Right or divisional application under ARTICLE V and such former Joint Patent Right or divisional application shall be deemed a 4DMT Patent Right; and (iii) uniQure shall and hereby does transfer and assign all right, title and interest in said former Joint Patent Right or of the divisional application to 4DMT as the sole owner. If 4DMT decides not to assume such responsibility, then it shall instruct Joint Counsel to abandon the Prosecution and Maintenance of such Joint Patent Right or not to file such divisional application.

(f) 4DMT shall notify uniQure and Joint Counsel at least [**] days in advance of the next deadline if (A) 4DMT decides that it does not wish to continue paying for the Prosecution and Maintenance of a particular Joint Patent Right for which no substitute has been filed, or (B) 4DMT decides that it intends to abandon claim scope in a Joint Patent Right which claim scope is intended to be maintained by uniQure, in which case, with respect to this clause (B), uniQure may assume responsibility for such claim scope by filing a divisional application restricted to such claim scope. In such cases (A) or (B), 4DMT shall allow uniQure to assume responsibility for Prosecution and Maintenance of the respective Patent Rights, including payments incurred after [**] days after receipt of 4DMT's notice. If uniQure assumes such responsibility, then: (i) uniQure may designate any counsel of its choice to handle the Prosecution and Maintenance of such Joint Patent Right or of the divisional application and it shall cease to be a part of the Joint Patent Rights and no further uniQure royalty obligations shall exist under this Agreement with respect thereto; (ii) 4DMT shall lose its licenses to such former Joint Patent Right or divisional application under ARTICLE V and such former Joint Patent Right or divisional application shall be deemed a uniQure Patent Right; and (iii) 4DMT shall and hereby does transfer and assign all right, title and interest in said former Joint Patent Right or of the divisional application to uniQure as the sole owner. If uniQure decides not to assume such responsibility, then it shall instruct Joint Counsel to abandon the Prosecution and Maintenance of such Joint Patent Right or not to file such divisional application.

7.6 Third Party Infringement.

(a) Notice. Each Party shall promptly report in writing to the other Party any known or suspected (i) infringement of any of the 4DMT Patent Rights, uniQure Patent Rights or Joint Patent Rights, or (ii) unauthorized use or misappropriation of any of the 4DMT Know-How, uniQure Know-How or Joint Know-How, of which such Party becomes aware and shall provide the other Party with all available evidence regarding such known or suspected infringement or unauthorized use.

(b) Enforcement of Solely Owned Patent Rights. uniQure shall have the sole right to enforce the uniQure Patent Rights, including the Core uniQure Patent Rights. Subject to UC's rights under the UCB Agreements with respect to any UC Patent Right included in the 4DMT Patent Rights, 4DMT shall have the sole right to enforce any 4DMT Patent Right,

including the Core 4DMT Patent Rights. Each Party shall cooperate in the prosecution of any such suit brought by the enforcing Party as may be reasonably requested by the enforcing Party; provided that the enforcing Party shall promptly reimburse all out-of-pocket expenses (including reasonable counsel fees and expenses) actually incurred by the non-enforcing Party in connection with such cooperation.

(c) Enforcement of Joint Patent Rights.

(i) In the Field. uniQure shall have the first right, but not the obligation, to initiate a lawsuit or take other reasonable action to enforce the Joint Patent Rights against any infringement in the Field. 4DMT shall cooperate in the prosecution of any such suit as may be reasonably requested by uniQure, including joining any action as party-plaintiff at uniQure's sole discretion; provided that uniQure shall promptly reimburse all out-of-pocket expenses (including reasonable counsel fees and expenses) actually incurred by 4DMT in connection with such cooperation.

(ii) Outside the Field. 4DMT shall retain any and all rights to initiate a lawsuit or take other reasonable action to enforce the Joint Patent Rights against any infringement outside the Field. uniQure shall cooperate in the prosecution of any such suit as may be reasonably requested by 4DMT, including joining any action as party-plaintiff at 4DMT's sole discretion; provided that 4DMT shall promptly reimburse all out-of-pocket expenses (including reasonable counsel fees and expenses) actually incurred by uniQure in connection with such cooperation.

(iii) Step-In Right. If either Party does not initiate a lawsuit or take other reasonable action pursuant to this Section 7.6(c) (the "Non-Enforcing Party"), then the other Party (the "Enforcing Party") shall have the right, but not the obligation, to initiate such lawsuit or take such other action, after providing [**] days' notice to the Non-Enforcing Party and giving good faith consideration to the Non-Enforcing Party's reason(s) for not initiating a lawsuit or taking other action. For this purpose, the Non-Enforcing Party shall cooperate in the prosecution of any such suit as may be reasonably requested by the Enforcing Party, including joining any action as party-plaintiff at the Non-Enforcing Party's sole discretion; provided, that the Enforcing Party shall promptly reimburse all out-of-pocket expenses (including reasonable counsel fees and expenses) actually incurred by the Non-Enforcing Party in connection with such cooperation.

(d) Conduct of Certain Actions; Costs. The Party initiating legal action shall have the sole and exclusive right to select counsel for any suit initiated by it pursuant to Section 7.6(b) or 7.6(c) (the "Initiating Party"). The Initiating Party shall bear its own out-of-pocket costs incurred in any such legal action, including the fees and expenses of the counsel selected by it. The other Party shall have the right to participate and be represented in any such legal action (in cases where such other Party has standing) by its own counsel at its own expense. The Initiating Party shall have the final say about the strategy and decisions in the suit and any settlement.

(e) Recoveries. Any amount recovered in any action or settlement of any such action shall be allocated first to equally reimburse each Party's actual out-of-pocket costs (including reasonable attorneys' fees and expenses) incurred in such action and any amount remaining shall be allocated to the Initiating Party; provided that if uniQure is the Initiating Party with respect to any such suit to enforce any Patent Right included in the Licensed IP in the Field, then, with respect to any remaining portion of such recovery, (i) any amount that reflects punitive or exemplary damages shall be allocated [**] percent ([**]%) to uniQure and [**] ([**]%) to 4DMT, and (ii) any other amounts shall be treated as Net Sales and subject to payment of royalties under Section 6.4(a); and provided further that if uniQure is the Initiating Party with respect to any such suit to enforce any Joint Patent Right outside the Field, or if 4DMT is the Initiating Party with respect to any such suit to enforce any Joint Patent Right in the Field, any amount remaining shall be allocated [**].

7.7 Patent Invalidity Claim. Each Party shall promptly notify the other in the event of any legal or administrative action by any Third Party against a 4DMT Patent Right, uniQure Patent Right or Joint Patent Right of which it becomes aware, including any nullity, revocation, reexamination or compulsory license proceeding. To the extent such action is in connection with an enforcement of such Patent Right under Section 7.6, the Parties' rights with respect to defending any such Patent Right in any such proceeding shall correspond to those set forth in Section 7.6.

7.8 Patent Term Extensions.

(a) uniQure shall have full and exclusive right to determine and control all filings of requests for any patent term extension or supplemental patent certificate or their equivalents in any country in the Territory for any uniQure Patent Right, including any Core uniQure Patent Right, and all costs and expenses relating thereto shall be paid by uniQure.

(b) 4DMT shall have full and exclusive right to determine and control all filings of requests for any patent term extension or supplemental patent certificate or their equivalents in any country in the Territory for any 4DMT Patent Right, including any Core 4DMT Patent Right, and all costs and expenses relating thereto shall be paid by 4DMT.

(c) The Parties shall jointly determine how to defend any such action relating to any Joint Patent Right.

(d) The Parties shall reasonably cooperate with each other in obtaining patent term extensions or supplemental protection certificates or their equivalents in any country in the Territory.

7.9 Orange Book; Paragraph IV Certification.

(a) uniQure shall have the right, but not the obligation, to list any uniQure Patent Rights in the then-current edition of the FDA publication "Approved Drug Products With Therapeutic Equivalence Evaluations" (the "Orange Book"), or equivalent patent listings in other countries.

(b) With respect to any notification provided by a Third Party to uniQure or 4DMT under 21 U.S.C. § 355(j)(2)(B) making a certification described in 21 U.S.C. § 355(j)(2)(A)(vii)(IV) with respect to any uniQure Patent Right that is listed for a Royalty Bearing Product in the Orange Book, or equivalent actions in other countries, (each a "Paragraph IV Certification"), the following shall apply notwithstanding Sections 7.6 and 7.7:

(i) Without any avoidable delay, however at the latest within [**] Business Days after receipt of any notification of a Paragraph IV Certification, such Party shall notify the other Party in writing and attach of copy of such notification. uniQure and 4DMT shall thereafter consult and cooperate fully to determine a course of action with respect to any such proceeding, including the negotiation of the offer of confidential access.

(ii) With respect to any uniQure Patent Right, uniQure shall have the sole right to initiate any infringement proceeding as a result of such Paragraph IV Certification (a "Paragraph IV Proceeding") with respect to a Royalty Bearing Product, including by commencing a patent infringement action under 35 U.S.C. § 271(e)(2)(A), and shall bear the expense of any such Paragraph IV Proceeding and, if legally required, may commence such action in 4DMT's or the relevant 4DMT Affiliate's name and on 4DMT's or the relevant 4DMT Affiliate's behalf.

(iii) Section 7.6(e) shall apply if any amount is recovered in any Paragraph IV Proceeding or settlement of any Paragraph IV Proceeding under this Section 7.9(b).

7.10 CREATE Act. Each Party acknowledges and agrees that this Agreement is a "joint research agreement" as contemplated by 35 U.S.C. § 102(c), and that all Inventions are intended to have the benefit of the rights and protections conferred by the Cooperative Research and Enhancement Act of 2004 (the "CREATE Act"). In the event that a Party seeks to rely on the foregoing and to invoke the CREATE Act with respect to any Invention, such Party will give prior written notice to the other Party of its intent to invoke the CREATE Act and of each submission or disclosure such Party intends to make to the United States Patent and Trademark Office (the "USPTO") pursuant to the CREATE Act, including: (a) any disclosure of the existence or contents of this Agreement to the USPTO, (b) the disclosure of any "subject matter developed by the other Party" (as such term is used in the CREATE Act) in an information disclosure statement or otherwise, or (c) the filing of any terminal disclaimer over the intellectual property of the other Party, it being agreed that no such submission, disclosure or filing shall be made by such Party without the prior written consent of the other Party, such consent not to be unreasonably withheld, conditioned or delayed, except that no such consent shall be required to disclose to the USPTO, through an information disclosure statement or otherwise, any "subject matter developed by the other Party" that was previously published or included in a published patent application by the other Party. The other Party will provide reasonable cooperation to such Party in connection with such Party's efforts to invoke and rely on the CREATE Act.

ARTICLE VIII

CONFIDENTIALITY AND PUBLICATION

8.1 Confidentiality Obligations. Each Party shall (a) maintain in confidence the Confidential Information of the other Party to the same extent such Party maintains its own confidential information, (b) not disclose such Confidential Information to any Third Party without the prior written consent of the other Party, and (c) not use such Confidential Information for any purpose except those permitted by this Agreement. Such obligations shall survive for a period of [**] years after termination or expiration of this Agreement, except that such obligations shall survive with respect to any Confidential Information identified by the disclosing Party as a trade secret for so long as such Confidential Information remains a trade secret.

8.2 Exceptions to Confidentiality. Notwithstanding the foregoing, the obligations of confidentiality set forth in Section 8.1 shall not apply to information that, in each case as demonstrated by competent written documentation:

(a) is publicly disclosed or made generally available to the public by the disclosing Party, either before or after it becomes known to the receiving Party;

(b) was known to the receiving Party, without any obligation to keep it confidential, prior to the date of first disclosure by the disclosing Party to the receiving Party, as shown by the receiving Party's files and records;

(c) is subsequently disclosed to the receiving Party by a Third Party lawfully in possession thereof without obligation to keep it confidential and without a breach of such Third Party's obligations of confidentiality;

(d) has been publicly disclosed or made generally available to the public other than through any act or omission of the receiving Party or its Affiliates in breach of this Agreement; or

(e) has been independently developed by the receiving Party without the aid, application or use of the disclosing Party's Confidential Information (the competent written proof of which must be contemporaneous with such independent development).

8.3 Authorized Disclosure. Notwithstanding Section 8.1, a Party may disclose Confidential Information of the other Party to the extent such disclosure is reasonably necessary in the following instances:

(a) Prosecuting and Maintaining Patent Rights in accordance with this Agreement;

(b) making filings with Regulatory Authorities in accordance with this Agreement;

(c) complying with applicable Laws or submitting information to tax or other Governmental Authorities; provided that if a Party is required by Law to make any public disclosure of Confidential Information of the other Party, to the extent it may legally do so, it will give reasonable advance notice to the other Party of such disclosure and will use its reasonable efforts to secure confidential treatment of such Confidential Information prior to its disclosure (whether through protective orders or otherwise);

(d) to its Affiliates, and to prospective and actual acquirers, licensees, sublicensees, employees, consultants, agents, accountants, lawyers, advisors, investors and underwriters, on a need to know basis, each of whom prior to disclosure must be bound by written or professional ethical obligations of confidentiality and non-use equivalent in scope to those set forth in this ARTICLE VIII and that are of reasonable duration in view of the circumstances of the disclosure; or

(e) to the extent mutually agreed to in writing by the Parties.

8.4 Scientific Publications. During the Research Term, neither Party shall first publish or first present in a public forum the scientific or technical results of any activity performed pursuant to this Agreement without the opportunity for prior review and comment by the other Party. Each Party agrees to provide the other Party with the opportunity to review any proposed abstract, manuscript or scientific presentation (including any verbal presentation) that relates to its activities performed pursuant to this Agreement during the Research Term, at least [**] days prior to its intended submission for publication and agrees, upon request, not to submit any such abstract or manuscript for publication until the other Party is given a reasonable period of time up to [**] months to secure patent protection for any material in such publication that it believes to be patentable. Both Parties understand that a reasonable commercial strategy may require delay of publication of information or filing of patent applications first with respect to activities performed or results obtained pursuant to this Agreement during the Research Term, or not to publish at all if necessary to preserve trade secrets. The Parties agree to review and decide whether to delay publication of such information to permit filing of patent applications. Neither Party shall have the right to publish or present any Confidential Information of the other Party, except as provided in Section 8.3. After the Research Term, each Party and its Affiliates may publish or present results, data or scientific findings of any of their activities performed after the Research Term without the prior review of the other Party, provided that such publication or presentation does not disclose any of the other Party's Confidential Information. After the Research Term, neither Party nor its Affiliates may publish or present any of the results, data or scientific findings of any activity performed by the other Party or its Affiliates pursuant to this Agreement without prior review and prior written consent of such other Party. Nothing contained in this Section 8.4 shall prohibit the inclusion of information necessary for a patent application; provided that the non-filing Party is given a reasonable opportunity to review the information to be included prior to submission of such patent application. For clarity, any publication under this Section 8.4 shall be consistent with uniQure's internal publication strategy, which shall be made available to 4DMT upon request. Nothing contained in this Section 8.4 shall prohibit either Party from disclosing the results, data or scientific findings of any activity performed by the other Party or its Affiliates pursuant to this Agreement without prior review and prior written consent of the other Party, where required, as reasonably

determined by the disclosing Party's legal counsel, by applicable Law; provided that if a Party is required by Law to make any such disclosure, to the extent it may legally do so, it will give reasonable advance notice to the other Party of such disclosure and will use its reasonable efforts to secure confidential treatment of such information prior to its disclosure (whether through protective orders or otherwise).

8.5 Press Releases and Other Permitted Disclosures.

(a) 4DMT and uniQure each agree not to disclose any of the terms and conditions of this Agreement to any Third Party, except as described below in this Section 8.5. The Parties will cooperate in the release of a mutually agreed upon press release announcing the collaboration contemplated

by this Agreement as soon as practicable after the Effective Date. Subject to the other provisions of this Agreement, no other press release, public statement or public disclosure concerning the existence or terms of this Agreement shall be made, either directly or indirectly, by either Party, without first obtaining the written approval of the other Party, which such approval shall not be unreasonably withheld or delayed beyond [**] Business Days (or [**] Business Days if the Party wishing to make such disclosure or any of its controlling Affiliates is then a public company) following submission to the approving Party of a draft of the respective press release, public statement or public disclosure. In no event shall any such subsequent press release, public statement or public disclosure by 4DMT disclose, if previously undisclosed, the identity of any Compound or Product or the stage of development of any Compound or Product that uniQure is researching, Developing, manufacturing, or Commercializing; provided that for clarity, uniQure may disclose, without the written approval of 4DMT, the identity of any Compound or Product or the stage of development of any Compound or Product that uniQure is researching, Developing, manufacturing, or Commercializing. In no event shall any such subsequent press release, public statement or public disclosure by a Party disclose, if previously undisclosed, the financial terms of this Agreement; provided that 4DMT may disclose the receipt of, and uniQure may disclose the payment of, any milestone payment but not the amount of such milestone payment; provided, further, however, that if disclosure of the amount of a milestone payment is required by applicable Law, by applicable stock exchange regulation, or by order or other ruling of a competent court, as set forth in Section 8.5(c), then 4DMT or uniQure, as the case may be, may also disclose such amount in a public statement or disclosure. Once any public statement or public disclosure has been approved in accordance with this Section 8.5, then either Party may appropriately communicate information contained in such permitted statement or disclosure.

(b) Either Party may disclose the existence and terms of this Agreement in confidence to its attorneys, to UC, and to each of the following, under an agreement with terms of confidentiality and non-use no less rigorous than the terms contained in this Agreement and, as applicable, to use such information solely for the purpose permitted pursuant to the applicable subsection of this Section 8.5(b):

(i) professional accountants, consultants, or auditors;

(ii) bankers or other financial advisors, in connection with an initial public offering, private financing or other strategic transaction, or corporate valuation for internal purposes;

(iii) potential acquirers (and their respective attorneys and professional advisors), in connection with a potential merger, acquisition or reorganization; provided that the Party making the disclosure has a *bona fide* offer (*e.g.*, a signed term sheet or letter of intent, even if non-binding) from such Third Party for such a transaction;

(iv) to actual or potential investors, lenders or permitted assignees of such Party (and their respective attorneys and professional advisors); or

(v) to actual or potential licensees or sublicensees of such Party (and their respective attorneys and professional advisors); provided that such disclosure in the case of 4DMT shall not include any financial terms, the Candidate Success Criteria, the Delivery Success Criteria, or Schedule 1.75.

(c) Notwithstanding the foregoing provisions of this ARTICLE VIII, a Party may disclose the existence and terms of this Agreement, however excluding, as far as legally possible, Schedule 1.75, or the Parties' activities under this Agreement, where required, as reasonably determined by the legal counsel of the disclosing Party, by applicable Law, by applicable stock exchange regulation or by order or other ruling of a competent court, although, to the extent practicable, the other Party shall be given [**] Business Days advance notice of any such legally required disclosure to comment and reasonably consider such comments provided by such other Party on the proposed disclosure. In case either Party is obliged to publish this Agreement as a "material agreement" in accordance with the U.S. stock exchange regulations ("SEC Filing"), this Agreement shall be redacted by the filing Party as far as legally possible, and the filing Party shall cooperate with the other Party reasonably in advance to such SEC Filing to enable the other Party to review and comment on the scope of such redaction.

ARTICLE IX

REPRESENTATIONS AND WARRANTIES; INDEMNIFICATION

9.1 Representations and Warranties of the Parties. uniQure and 4DMT each represent, warrant and covenant to the other that:

(a) subject to approval by uniQure B.V.'s Class A shareholders of uniQure's entry into this Agreement, as of the Effective Date, it has the authority and right to enter into and perform this Agreement and grant the rights embodied herein, and it is not aware of any legal impediment that could inhibit its ability to perform its obligations under this Agreement;

(b) as of the Effective Date, its execution, delivery and performance of this Agreement does not conflict with, or constitute a breach of, any order, judgment, agreement or instrument to which it is a party or is otherwise bound;

(c) it shall comply in all material respects with all Laws applicable to its actions under this Agreement; and

(d) as of the Effective Date, no consent of any Third Party is required for such Party to grant the licenses and rights granted to the other Party under this Agreement or to perform its obligations hereunder.

9.2 Representations and Warranties of 4DMT. 4DMT represents, warrants and covenants to uniQure that:

(a) as of the Effective Date, Schedule 1.5 is compiled accurately and, to the extent set forth in Section 1.5, is complete regarding the subject matter set forth therein;

(b) as of the Effective Date, 4DMT has not previously assigned, transferred, conveyed or otherwise encumbered its right, title and interest in 4DMT Intellectual Property in a manner inconsistent with the terms hereof;

(c) as of the Effective Date, 4DMT has valid and existing licenses, free and clear of all liens, charges and encumbrances, to the 4DMT Patent Rights not owned by 4DMT;

(d) as of the Effective Date, to 4DMT's knowledge, the conception, development and reduction to practice of the 4DMT Intellectual Property has not constituted or involved the misappropriation of trade secrets of any Third Party or the infringement of issued Patent Rights of any Third Party;

(e) as of the Effective Date, 4DMT has not received any written notice of any unauthorized use, infringement, or misappropriation by any person or entity, including any current or former employee or consultant of 4DMT, of any 4DMT Intellectual Property;

(f) as of the Effective Date, to 4DMT's knowledge, there are no claims, judgments, settlements pending or any action with respect to the 4DMT Intellectual Property;

(g) as of the Effective Date, to 4DMT's knowledge, uniQure's use of the 4DMT Intellectual Property, as reasonably anticipated to be used in the conduct of the Research Program, will not infringe any valid Patent Right existing as of the Effective Date and owned by any Third Party;

(h) all of 4DMT's personnel and employees, and Third Parties, including agents and consultants, hired by 4DMT and involved in the Research Program are, or when hired will be, under a written obligation to assign to 4DMT any right they may have in any Invention first invented, discovered, made, conceived or reduced to practice in the conduct of activities pursuant to the Research Program, and all intellectual property rights therein;

(i) it will not, after the Effective Date, enter into any written or oral contractual obligation with any Third Party that would be inconsistent with the obligations that

arise on its part out of this Agreement or that would deprive uniQure of the benefits of or rights granted under this Agreement;

(j) as of the Effective Date, each of the UCB Agreements is in full force and effect, and 4DMT will not, after the Effective Date, terminate, amend or otherwise modify any of the terms thereof without prior written consent from uniQure, or take any action or refrain from taking any action that would permit UC to terminate any UCB Agreement (it being recognized that if the Selected Capsid Variants are not UC AAV Capsid Variants, and UC terminates any UCB Agreement, 4DMT shall not be deemed to be in breach of the foregoing), and 4DMT shall promptly provide uniQure with a copy of each notice it receives from UC under any UCB Agreement; and

(k) if, during the Term, 4DMT has reason to believe that it or any of its employees, officers, subcontractors, or consultants rendering services hereunder (i) is or shall be debarred or convicted of a crime under 21 U.S.C. Section 335a, or (ii) is or shall be under indictment under said Section 335a, then 4DMT shall immediately notify uniQure in writing.

For purposes of this Section 9.2, "knowledge" shall mean the actual knowledge of 4DMT, including David Schaffer and David Kirn.

9.3 Representations and Warranties of uniQure. uniQure represents, warrants and covenants to 4DMT that:

(a) all of uniQure's personnel and employees, and Third Parties, including agents and consultants, hired by uniQure and involved in the Research Program are, or when hired will be, under a written obligation to assign to uniQure any right they may have in any Invention first invented, discovered, made, conceived or reduced to practice in the conduct of activities pursuant to the Research Program, and all intellectual property rights therein;

(b) it will not, after the Effective Date, enter into any written or oral contractual obligation with any Third Party that would be inconsistent with the obligations that arise on its part out of this Agreement or that would deprive 4DMT of the benefits of or rights granted under this Agreement;

(c) if, during the Term, uniQure has reason to believe that it or any of its employees, officers, subcontractors, or consultants rendering services hereunder (i) is or shall be debarred or convicted of a crime under 21 U.S.C. Section 335a, or (ii) is or shall be under indictment under said Section 335a, then uniQure shall immediately notify 4DMT in writing.

9.4 No Other Warranties.

(a) EXCEPT AS OTHERWISE EXPRESSLY SET FORTH HEREIN, THE PARTIES MAKE NO REPRESENTATIONS AND EXTEND NO WARRANTIES OF ANY KIND, EITHER EXPRESS OR IMPLIED, AND PARTICULARLY THAT PRODUCT(S) WILL BE SUCCESSFULLY DEVELOPED HEREUNDER, AND IF PRODUCT(S) ARE DEVELOPED, WITH RESPECT TO SUCH PRODUCT(S), THE

PARTIES DISCLAIM ALL IMPLIED WARRANTIES OF TITLE, NON-INFRINGEMENT, MERCHANTABILITY AND FITNESS FOR A PARTICULAR PURPOSE.

(b) uniQure acknowledges that UC has not warranted to 4DMT under the UCB Agreements as to the validity of any Patent Rights or that practice under such Patent Rights shall be free of infringement. UNIQURE, ITS AFFILIATES AND ITS SUBLICENSEE(S) AGREE THAT (I) THE LICENSES GRANTED PURSUANT TO THE UCB AGREEMENTS, THE UC AAV CAPSID VARIANTS, AND THE ASSOCIATED INVENTIONS ARE PROVIDED WITHOUT WARRANTY OF MERCHANTABILITY OR FITNESS FOR A PARTICULAR PURPOSE OR ANY OTHER WARRANTY, EXPRESSED OR IMPLIED; (II) UC MAKES NO REPRESENTATION OR WARRANTY THAT ANY INVENTION CLAIMED BY THE UC PATENT RIGHTS, THE UC AAV CAPSID VARIANTS, THE UC PATENT RIGHTS, OR THE UC PRODUCTS WILL NOT INFRINGE ANY PATENT OR OTHER PROPRIETARY RIGHT; AND (III) IN NO EVENT WILL UC BE LIABLE FOR ANY INCIDENTAL, SPECIAL OR CONSEQUENTIAL DAMAGES RESULTING FROM EXERCISE OF THE LICENSES GRANTED PURSUANT TO THE UCB AGREEMENTS OR THE USE OF ANY INVENTION CLAIMED BY THE UC PATENT RIGHTS, THE UC AAV CAPSID VARIANTS, THE UC PATENT RIGHTS, OR THE UC PRODUCTS.

9.5 Indemnification by uniQure. uniQure shall indemnify, hold harmless and defend 4DMT, its Affiliates and all of their respective officers, directors, employees, agents and shareholders (collectively, the "4DMT Indemnitees") from and against any and all losses, damages, liabilities, judgments, fines, amounts paid in settlement, expenses and costs of defense (including reasonable attorneys' fees and witness fees) (collectively, "Damages") resulting from any demand, claim, action or proceeding brought or initiated by a Third Party (each a "Third Party Claim") against any 4DMT Indemnitee to the extent arising out of: (a) a Default by uniQure; (b) the negligence or willful misconduct of a uniQure Indemnitee; or (c) the use, Development, Commercialization, storage or other exploitation of any Compound or Product by uniQure, its Affiliates, Sublicensees, Third Party Distributors, or Third Party independent contractors; provided that (i) the 4DMT Indemnitees shall comply with the procedures set forth in Section 9.7(a); and (ii) such indemnity shall not apply to the extent such Third Party Claim is subject to indemnification by 4DMT under Section 9.6.

9.6 Indemnification by 4DMT. 4DMT shall indemnify, hold harmless and defend uniQure, its Affiliates and all of their respective officers, directors, employees, agents, and shareholders (collectively, the "uniQure Indemnitees") from and against any and all Damages resulting from any Third Party Claim against any uniQure Indemnitee to the extent arising out of: (a) a Default by 4DMT; (b) the negligence or willful misconduct of a 4DMT Indemnitee; or (c) the use, Development, Commercialization, storage or other exploitation of any 4DMT AAV Capsid Vector, Compound or Product (other than a Royalty Bearing Compound or Royalty Bearing Product) by 4DMT, its Affiliates, sublicensees or Third Party independent contractors; provided that (i) the uniQure Indemnitees shall comply with the procedures set forth in Section 9.7(b); and (ii) such indemnity shall not apply to the extent such Third Party Claim is subject to indemnification by uniQure under Section 9.5.

9.7 Procedure.

(a) To be eligible for the 4DMT Indemnitees to be indemnified hereunder, 4DMT shall provide uniQure with prompt notice of the Third Party Claim giving rise to the indemnification obligation under Section 9.5 and the exclusive ability to defend or settle any such claim; provided however that uniQure shall not enter into any settlement for damages, or that imposes upon 4DMT any obligation or liability, without 4DMT's prior written consent, such consent not to be unreasonably withheld, delayed or conditioned. 4DMT shall have the right to participate, at its own expense and with counsel of its choice, in the defense of any claim or suit that has been assumed by uniQure.

(b) To be eligible for the uniQure Indemnitees to be indemnified hereunder, uniQure shall provide 4DMT with prompt notice of the Third Party Claim giving rise to the indemnification obligation under Section 9.6 and the exclusive ability to defend or settle any such claim; provided however that 4DMT shall not enter into any settlement for damages, or that imposes upon uniQure any obligation or liability, without uniQure's prior written consent, such consent not to be unreasonably withheld, delayed or conditioned. uniQure shall have the right to participate, at its own expense and with counsel of its choice, in the defense of any claim or suit that has been assumed by 4DMT.

9.8 uniQure Indemnity to UC. uniQure shall, and shall require its Sublicensees to, indemnify, defend, and hold harmless UC and IGT, and their officers, employees, and agents; sponsor(s) of the research that led to the inventions disclosed in the UC Patent Rights and the UC AAV Capsid Variants; and the inventors of any UC Patent Rights and their employers against any and all losses, damages, costs, fees, and expenses resulting from Third Party claims and suits arising out of uniQure's activities under this Agreement or of any Sublicensee activities under any sublicense agreement granting rights under the UC Patent Rights or the UC AAV Capsid Variants, or any use or possession of the UC AAV Capsid Variants resulting from uniQure's exploitation of its rights thereto. This indemnification will include any product liability claims. uniQure will keep UC informed of its defense of any claims pursuant to this Section 9.8, and UC will cooperate reasonably in any such suit. If UC invokes the provisions of this Section 9.8, UC will not make any admissions or take any actions in such claim or suit that may prejudice or impair uniQure's ability to defend such claim or suit without uniQure's prior written consent, and uniQure will not admit liability or wrongdoing on behalf of UC without UC's prior written consent.

9.9 Insurance. Each Party shall procure and maintain insurance or self-insurance, including general liability insurance and product liability insurance, adequate to cover its obligations hereunder and that are consistent with normal business practices of prudent companies similarly situated, at all times during which any Research Compound, Royalty Bearing Compound, or Royalty Bearing Product is being Developed, clinically tested in human subjects or Commercialized by or on behalf of such Party, its Affiliates or sublicensees, including, in the case of uniQure, its Sublicensees. It is understood that any such insurance or self-insurance shall not be construed to create a limit of a Party's liability with respect to its indemnification obligations under this ARTICLE IX. Each Party shall provide the other Party with written evidence of such insurance or self-insurance upon request. Each Party shall provide

the other Party with written notice at least [**] days prior to the cancellation, non-renewal or material change in such insurance or self-insurance which could adversely affect rights hereunder. Without limiting the generality of the foregoing:

(a) uniQure, at its sole cost and expense, will ensure that the applicable entity performing activities in connection with any work performed hereunder, whether uniQure, an Affiliate, or a Sublicensee, will obtain, keep in force, and maintain the following insurance:

(i) prior to the start of Clinical Trials of a UC Product, commercial form general liability insurance (contractual liability included) with limits as follows:

Each Occurrence	[**]
Products/Completed Operations Aggregate	[**]
Personal and Advertising Injury	[**]
General Aggregate	[**]

(ii) Upon the start of any Clinical Trials of a UC Product, commercial form general liability insurance (contractual liability included), and product liability insurance if not otherwise included, with limits as follows:

Each Occurrence	[**]
Products/Completed Operations Aggregate	[**]
Personal and Advertising Injury	[**]
General Aggregate	[**]

(iii) upon the First Commercial Sale of a UC Product, commercial form general liability insurance (contractual liability included), and product liability insurance if not otherwise included, with limits as follows:

Each Occurrence	[**]
Products/Completed Operations Aggregate	[**]
Personal and Advertising Injury	[**]
General Aggregate	[**]

If the above insurance is written on a claims-made form, it shall continue for [**] years following termination or expiration of this Agreement.

(iv) worker's compensation as legally required in the jurisdiction in which uniQure, an Affiliate, or a Sublicensee, as applicable, is doing business.

uniQure will promptly notify UC of any material reduction in the insurance coverages below the amounts required hereunder.

(b) Within [**] days after the Effective Date, uniQure will furnish 4DMT with certificates of insurance evidencing compliance with all requirements. Such certificates will:

(i) where possible, provide for [**] days' ([**] days for non-payment of premium) advance written notice to 4DMT and UC of any cancellation of insurance coverages described above in Section 9.9(a);

(ii) indicate that 4DMT and UC have been endorsed as additional insureds under the coverage described above in Section 9.9(a); and

(iii) include a provision that the coverages described above in Section 9.9(a) will be primary and will not participate with, nor will be excess over, any valid and collectable insurance or program of self-insurance maintained by 4DMT or UC.

9.10 No Consequential or Punitive Damages. EXCEPT WITH RESPECT TO (a) THE INDEMNIFICATION RIGHTS OR OBLIGATIONS OF EITHER PARTY UNDER THIS AGREEMENT WITH RESPECT TO THIRD PARTY CLAIMS, (b) A BREACH OF THE CONFIDENTIALITY OBLIGATIONS OF ARTICLE VIII, (c) A BREACH OF SECTION 5.6, OR (d) A PARTY'S WILLFUL MISCONDUCT, NEITHER PARTY HERETO WILL BE LIABLE FOR INDIRECT, INCIDENTAL, CONSEQUENTIAL, SPECIAL, EXEMPLARY OR PUNITIVE DAMAGES, INCLUDING LOST PROFITS, ARISING FROM OR RELATING TO THIS AGREEMENT, REGARDLESS OF ANY NOTICE OF SUCH DAMAGES.

ARTICLE X

TERM AND TERMINATION

10.1 Term and Expiration. This Agreement shall be effective as of the Effective Date and unless terminated earlier pursuant to Section 10.2, this Agreement shall continue in effect until the expiration of all of uniQure's payment obligations hereunder (the "Term"). Upon expiration, all licenses granted hereunder shall be fully paid-up, perpetual and irrevocable. Notwithstanding the foregoing, this Agreement shall be void *ab initio* if uniQure B.V.'s Class A shareholders have not approved uniQure's entry into this Agreement and the Grant Letters by January 31, 2014, or if David Schaffer has not been elected to the Supervisory Board of uniQure B.V. by January 31, 2014.

10.2 Termination.

(a) Termination of Agreement for Cause.

(i) This Agreement may be terminated at any time during the Term upon written notice by either Party (the "Non-Defaulting Party") upon Default of the other

Party (the "Defaulting Party"), which Default remains uncured for [**] days after written notice requesting cure of such Default. The Non-Defaulting Party shall provide written notice to the Defaulting Party, which notice shall identify the Default, the intent to so terminate and the actions or conduct that it considers would be an acceptable cure of such Default. If the Defaulting Party disputes the Default under this Section 10.2(a), then the issue of whether the Non-Defaulting Party may properly terminate this Agreement on expiration of the applicable cure period shall be resolved in accordance with ARTICLE XI. If, as a result of such dispute resolution process, it is determined that the alleged Defaulting Party committed a Default and the Defaulting Party does not cure such Default within [**] days after the date of such dispute resolution award (the "Additional Cure Period"), then such termination shall be effective as of the expiration of the Additional Cure Period. If the Parties dispute whether such Default was so cured, either Party alone may request the same tribunal to determine whether it was so cured, and the Parties shall cooperate to allow such determination to be made within [**] days after such request by either Party. Any such dispute resolution proceeding does not suspend any obligation of either Party hereunder, and each Party shall use reasonable efforts to mitigate any damage. If, as a result of any such dispute resolution proceeding, it is determined that the alleged Defaulting Party did not commit such Default (or such Default was cured in accordance with this Section 10.2(a)), then no termination shall be effective, and this Agreement shall continue in full force and effect. Notwithstanding the foregoing, if 4DMT is the Non-Defaulting Party and the claimed Default by uniQure as the Defaulting Party relates to one or more Compounds or Products, and not this entire Agreement, then this Agreement shall be terminated only with respect to the Indication for which such Compound(s) or Product(s) were intended to treat and such Indication shall be removed from the Field.

(ii) Notwithstanding Section 10.2(a)(i), uniQure shall have the right to terminate this Agreement during the Research Term immediately upon written notice to 4DMT if David Schaffer ceases to be a representative of 4DMT on the JRSC or is otherwise unavailable to direct 4DMT's Research Program activities during any consecutive fifteen (15) Business Day period, in each case for any reason other than his death, illness or disability, which shall be deemed a Default by 4DMT.

(b) Termination for Bankruptcy. To the extent allowed under applicable Law, either Party shall have the right to terminate this Agreement in the event of the commencement of any proceeding in or for bankruptcy, insolvency, dissolution or winding up by or against the other Party (other than pursuant to a corporate restructuring) that is not dismissed or otherwise disposed of within sixty (60) days thereafter.

(c) Termination for Futility. uniQure shall have the right terminate this Agreement immediately upon written notice to 4DMT summarizing the basis for such termination if, at any point prior to the first (1st) anniversary of the Effective Date, the JRSC determines that (i) it would be futile to continue the Research Program, including if the JRSC determines that any Candidate Success Criteria or Delivery Success Criteria cannot be met through use of the 4DMT Intellectual Property following the reasonable efforts of 4DMT to achieve such Candidate Success Criteria or Delivery Success Criteria or (ii) 4DMT is not making *bona fide* efforts to achieve the timelines set forth in the Research Plan.

(d) Termination for Convenience. uniQure shall have the right terminate this Agreement at any time after the Research Term, for any reason or for no reason, by giving 4DMT ninety (90) days' prior written notice thereof.

(e) Special Termination Right of 4DMT. In the event that (i) uniQure B.V. does not complete an underwritten public offering of its ordinary shares pursuant to an effective registration statement under the U.S. Securities Act of 1933 and the listing of its ordinary shares on the Nasdaq Global Market by September 1, 2014, December 31, 2014, or December 31, 2015, as the case may be, and (ii) uniQure B.V. has not agreed in writing to pay the applicable "Cash-Out Amount" provided for in Article 4c of each of the Grant Letters in respect of options that will vest on the first vesting date following such applicable date, 4DMT shall have the right to terminate this Agreement by providing written notice thereof to uniQure within thirty (30) days following such applicable date, and any such termination shall be effective as of the thirtieth (30th) day following such applicable date.

10.3 Effect of Termination

(a) If uniQure terminates this Agreement under Section 10.2(a) or Section 10.2(b):

(i) uniQure's licenses pursuant to this Agreement shall continue; provided however that uniQure shall continue to fulfill uniQure's payment obligations with respect to milestones and royalties under ARTICLE VI; and provided further that uniQure may reduce such payment obligations by the amount of monetary damage suffered by uniQure as a direct result of 4DMT's Default, as determined (A) in a final decision of the arbitrators in accordance with Section 11.2 or, with respect to an Excluded Claim, a court of competent jurisdiction, which decision is not appealable or has not been appealed within the time allowed for appeal, or (B) by the Parties in a settlement agreement;

(ii) 4DMT shall, within [**] days after the effective date of such termination, return or cause to be returned to uniQure, copies of all uniQure's Confidential Information and uniQure Intellectual Property and all Materials provided by uniQure, except that 4DMT may retain one copy of uniQure's Confidential Information solely for legal archive purposes and to exercise the licenses granted to 4DMT which survive termination of this Agreement;

(iii) For clarity, uniQure shall be released of its ongoing diligence obligations under Section 4.2 and uniQure and 4DMT shall be released of their disclosure and information exchange obligations under ARTICLE III and ARTICLE IV;

(iv) For clarity, the JRSC and its subcommittees shall not meet anymore;

(v) No further options under each Grant Letter shall vest from and after the effective date of such termination; and

(vi) If this Agreement is terminated pursuant to Section 10.2(a)(ii), uniQure shall continue to fund the FTEs included in the Research Plan pursuant to Section 6.2(b) for the [**] months immediately following the effective date of such termination.

(b) Upon termination of this Agreement by uniQure under Section 10.2(c) or Section 10.2(d), or by 4DMT under Section 10.2(a), Section 10.2(b), or Section 10.2(e):

(i) For clarity, uniQure's licenses pursuant to Section 5.1 and 4DMT's exclusivity obligations pursuant to Section 5.6 shall terminate as of the effective date of such termination;

(ii) Effective as of the effective date of such termination, the license granted to 4DMT under Section 5.2(b) shall be automatically expanded to include the Selected Capsid Variants and all fields of use;

(iii) uniQure shall, within [**] days after the effective date of such termination, return or cause to be returned to 4DMT, copies of all 4DMT's Confidential Information and 4DMT Intellectual Property and all Materials provided by 4DMT; except that uniQure may retain one copy of the 4DMT Confidential Information solely for legal archive purposes;

(iv) 4DMT shall, within [**] days after the effective date of such termination, return or cause to be returned to uniQure, copies of all uniQure's Confidential Information and uniQure Intellectual Property and all Materials provided by uniQure, except that 4DMT may retain one copy of uniQure's Confidential Information solely for legal archive purposes and to exercise the licenses granted to 4DMT which survive termination or are granted upon termination of this Agreement;

(v) For a period of [**] months, if termination occurs after Regulatory Approval of Royalty Bearing Products, uniQure and its Affiliates shall be entitled to finish work in progress and to sell any of the Royalty Bearing Products remaining in inventory in accordance with the terms of this Agreement to the extent such Royalty Bearing Products were being sold in the Territory at the time of termination, provided that such sales shall be subject to the royalty and milestone provisions of this Agreement;

(vi) If this Agreement is terminated pursuant to Section 10.2(c), (A) uniQure shall continue to fund the FTEs included in the Research Plan pursuant to Section 6.2(b) for the [**] months immediately following the effective date of such termination, but in no event for less than [**]

after the Effective Date, and (B) no further options under each Grant Letter shall vest from and after the date that [**] percent ([**]%) of all options under such Grant Letter have vested; and

(vii) If this Agreement is terminated pursuant to Section 10.2(e), uniQure shall continue to fund the FTEs included in the Research Plan pursuant to Section 6.2(b) for the [**] months immediately following the effective date of such termination, but in no event for less than [**] after the Effective Date.

Notwithstanding the foregoing, if such termination is under Section 10.2(a) solely with respect to one or more given Indication(s), then uniQure's licenses pursuant to Section 5.1 do not terminate but the Field is automatically narrowed to exclude the relevant Indication(s), and 4DMT's exclusivity obligations pursuant to Section 5.6 terminate solely with respect to the relevant Indications; subsection (ii) shall apply; the license granted to 4DMT under Section 5.2(b) shall be automatically expanded to include the relevant Indication(s) rather than all fields of use; and uniQure's obligations under subsection (iii) shall be limited to copies of 4DMT's Confidential Information and 4DMT Intellectual Property and Materials that relate solely to the relevant Indication(s).

10.4 Effect of Expiration or Termination; Survival.

(a) Expiration or termination of this Agreement shall not relieve the Parties of any obligation accruing prior to such expiration or termination. Any expiration or termination of this Agreement shall be without prejudice to the rights of either Party against the other accrued or accruing under this Agreement prior to expiration or termination, including the obligation to pay royalties for Royalty Bearing Product(s) sold prior to such expiration or termination. 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.

(b) The provisions of ARTICLE I, ARTICLE VII, ARTICLE VIII, ARTICLE XI, ARTICLE XII, and Sections 4.5, 5.2(b), 5.4, 5.5, 6.2(c), 9.4, 9.5, 9.6, 9.7, 9.8, 9.9, 9.10, 10.3 and 10.4 shall survive any expiration or termination of this Agreement, and with respect to those Royalty Bearing Products in such countries for which uniQure retains a Development and Commercialization license after the expiration or termination of this Agreement, the provisions of ARTICLE VI shall also survive.

ARTICLE XI

DISPUTE RESOLUTION

11.1 Seeking Consensus. If any dispute arises out of, in connection with or related to this Agreement, including disputes over the interpretation, performance, enforcement or breach of this Agreement, including any dispute that is not within the jurisdiction of the JRSC, (a "Dispute"), excluding any dispute resolved in accordance with Section 2.5(c) (subject to Section 2.5(d)), then upon the written request of either Party, the matter shall be referred to the Executives, who shall meet in a good faith effort to resolve the dispute within [**] days. If the Parties' Executives cannot agree on a resolution of the Dispute within such [**] day period, then it shall be resolved pursuant to the remaining provisions of this ARTICLE XI.

11.2 Arbitration. If the Parties do not fully settle a Dispute pursuant to Section 2.5 (only as to those matters that may be referred to arbitration) or 11.1, as applicable, and a Party wishes to pursue the matter, each such Dispute that is not an Excluded Claim (as defined below) shall be finally resolved by binding arbitration in accordance with the Rules of

Arbitration of the ICC (International Chamber of Commerce) and judgment on the arbitration award may be entered in any court having jurisdiction thereof.

(a) The arbitration shall be conducted by a panel of three (3) persons. Within [**] days after initiation of arbitration, each Party shall select one person to act as arbitrator and the two Party-selected arbitrators shall select a third arbitrator within [**] days after their appointment. If the arbitrators selected by the Parties are unable or fail to agree upon the third arbitrator, the third arbitrator shall be appointed by the ICC. The place of arbitration shall be New York City, New York, and all proceedings and communications shall be in English.

(b) Either Party may apply to the arbitrators for interim injunctive relief until the arbitration award is rendered or the Dispute is otherwise resolved. Either Party also may, without waiving any remedy under this Agreement, seek from any court having jurisdiction any injunctive or provisional relief necessary to protect the rights or property of that Party pending the arbitration award. The scope of the authority of the arbitrators shall be limited to the strict application of law. The arbitrators shall have no authority to award punitive or any other type of damages not measured by a Party's compensatory damages, except as permitted by Section 9.10. Each Party participating in an arbitration pursuant to the terms of this Agreement shall, subject to the award of the arbitrators, pay an equal share of the arbitrators' fees. The arbitrators shall have the power to award recovery of all costs (including reasonable attorney's fees, administrative fees, arbitrators' fees and court costs) to the prevailing Party.

(c) Neither Party shall be required to give general discovery of documents, but may be required to produce documents or testimony that are relevant or considered relevant by the arbitrators to the Dispute. It is the objective and intent of the Parties that any arbitration proceeding be conducted in such a manner that a decision will be rendered by the arbitrators within [**] days after the third arbitrator is appointed to the panel, and the Parties and the panel selected in the manner provided above will adopt rules and procedures intended to implement such objective and intent.

(d) Except to the extent necessary to confirm or vacate an award or as may be required by Law (including applicable securities laws or the rules of any stock exchange on which a Party's securities may then be listed), neither a Party nor an arbitrator may disclose the existence, content, or results of arbitration without the prior written consent of both Parties. In no event shall arbitration be initiated after the date when commencement of a legal or equitable proceeding based on the dispute, controversy or claim would be barred by the applicable New York statute of limitations.

(e) The Parties agree that any payment made pursuant to this Agreement pending resolution of the Dispute shall be refunded or credited if the arbitrators or court determines that such payments are not due.

As used in this Section 11.2, the term "Excluded Claim" shall mean a Dispute that concerns (a) the validity, enforceability, scope or infringement of a patent, trademark or copyright; or (b) any antitrust, anti-monopoly or competition law or regulation, whether or not statutory.

ARTICLE XII

MISCELLANEOUS

12.1 Governing Law. This Agreement shall be governed by and construed in accordance with the Laws of the State of New York, other than any principle of conflict or choice of laws that would cause the application of the Laws of any other jurisdiction.

12.2 Waiver. Waiver by a Party of a breach hereunder by the other Party shall not be construed as a waiver of any succeeding breach of the same or any other provision. No delay or omission by a Party to exercise or avail itself of any right, power or privilege that it has or may have hereunder shall operate as a waiver of any right, power or privilege by such Party. No waiver shall be effective unless made in writing with specific reference to the relevant provision(s) of this Agreement and signed by a duly authorized representative of the Party granting the waiver.

12.3 Notices. All notices, instructions and other communications hereunder or in connection herewith shall be in writing, shall be sent to the address specified in this Section 12.3 and shall be: (a) delivered personally; (b) transmitted by facsimile; (c) sent by registered or certified mail, return receipt requested, postage prepaid; or (d) sent via a reputable international overnight delivery service. Any such notice, instruction or communication shall be deemed to have been delivered (i) upon receipt if delivered by hand, (ii) when transmitted with electronic confirmation of receipt, if transmitted by facsimile (if such transmission is on a Business Day; otherwise, on the next Business Day following such transmission), provided that an original document is sent via an internationally recognized overnight delivery service (receipt requested), (iii) three (3) Business Days after it is sent by registered or certified mail, return receipt requested, postage prepaid, or (iv) one (1) Business Day after it is sent via a reputable international overnight delivery service.

If to 4DMT, to:
4D Molecular Therapeutics, LLC
19 Rima Court
Danville, CA 94526
Attention: David Schaffer
Facsimile: (650) 463-2600

with a copy to:
Latham & Watkins LLP
140 Scott Drive
Menlo Park, CA 94025
Attention: Alan Mendelson and Judith Hasko
Facsimile: (650) 463-2600

If to uniQure, to:
uniQure biopharma B.V.
P.O. Box 22506
1100 DA Amsterdam
The Netherlands
Attention: CEO
Facsimile: +31 20 566 9272

with a copy to:
Wilmer Cutler Pickering Hale and Dorr LLP
60 State Street
Boston, MA 02109 USA
Attention: David Redlick
Facsimile: (617) 526-5000

or to such other address as the Party to whom notice is to be given may have furnished to the other Party in writing in accordance herewith.

12.4 Entire Agreement; Amendment. This Agreement (including its Exhibits and Schedules) contains the complete understanding of the Parties with respect to the subject matter hereof and supersedes all prior understandings and writings relating to such subject matter. In particular, it supersedes and replaces the Prior Confidentiality Agreement and any and all term sheets relating to the transactions contemplated by this Agreement and exchanged between the Parties or their Affiliates prior to the Effective Date. No amendment, change or addition to this Agreement will be effective or binding on either Party unless reduced to writing and duly executed on behalf of both Parties.

12.5 Headings. Headings in this Agreement are for convenience of reference only and shall not be considered in construing this Agreement.

12.6 Severability. If any provision or portion thereof in this Agreement is for any reason held to be invalid, illegal or unenforceable, the same shall not affect any other portion of this Agreement, as it is the intent of the Parties that this Agreement shall be construed in such fashion as to maintain its existence, validity and enforceability to the greatest extent possible. In any such event, this Agreement shall be construed as if such clause of portion thereof had never been contained in this Agreement, and there shall be deemed substituted therefor such provision as will most nearly carry out the intent of the Parties as expressed in this Agreement to the fullest extent permitted by applicable Law.

12.7 Assignment. Neither this Agreement nor any right or obligation hereunder may be assigned or otherwise transferred by any Party without the consent of the other Party; provided, however, that any Party may, without such consent, assign this Agreement, in whole or in part: (a) to any of its respective Affiliates; provided that the assigning Party shall remain jointly and severally liable with such Affiliate in respect of all obligations so assigned, or (b) 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 (an "M&A Event"). Any assignment not in accordance with this Section 12.7 shall be void. Each Party agrees that, notwithstanding any provision of this Agreement to the contrary, neither the assignment of this Agreement by a Party in

connection with an M&A Event, nor the occurrence of such M&A Event (whether or not a formal assignment of this Agreement occurs), shall provide the non-assigning Party with rights or access to any intellectual property or technology of the acquirer of the assigning Party or its Affiliates that were not Affiliates of the assigning Party prior to such M&A Event. If uniQure assigns its rights and obligations hereunder to an Affiliate or Third Party outside the United States or The Netherlands pursuant to this Section 12.7, and if such Affiliate or Third Party shall be required by applicable Law to withhold additional taxes from or in respect of any amount payable under this Agreement as a result of such assignment, then any such amount payable under this Agreement shall be increased to take into account the additional taxes withheld as may be necessary so that, after making all required withholdings, 4DMT receives an amount equal to the sum it would have received had no such assignment been made.

12.8 Counterparts. This Agreement may be executed in any number of counterparts, each of which shall be deemed an original but all of which together shall constitute one and the same instrument. Signatures provided by facsimile transmission or in Adobe Portable Document Format (PDF) sent by electronic mail shall be deemed to be original signatures.

12.9 Force Majeure. No Party shall be liable for failure of or delay in performing obligations (other than payment obligations) set forth in this Agreement, and no Party shall be deemed in breach of its obligations, if such failure or delay is due to a natural disaster, explosion, fire, flood, tornado, thunderstorm, hurricane, earthquake, war, terrorism, riot, embargo, loss or shortage of power, labor stoppage, substance or material shortage, events caused by reason of laws of any Governmental Authority, events caused by acts or omissions of a Third Party or any other cause reasonably beyond the control of such Party, if the Party affected gives prompt notice of any such cause to the other Party. The Party giving such notice shall thereupon be excused from such of its obligations hereunder as it is thereby disabled from performing for so long as it is so disabled, provided, however, that such affected Party commences and continues to use its Commercially Reasonable Efforts to cure such cause.

12.10 Third Party Beneficiaries. None of the provisions of this Agreement shall be for the benefit of or enforceable by any Third Party, other than a 4DMT Indemnitee under Section 9.5 or uniQure Indemnitee under Section 9.6. No such Third Party shall obtain any right under any provision of this Agreement or shall by reason of any such provision make any claim in respect of any debt, liability or obligation (or otherwise) against either Party.

12.11 Relationship of the Parties. Each Party shall bear its own costs incurred in the performance of its obligations hereunder without charge or expense to the other, except as expressly provided in this Agreement. Neither Party shall have any responsibility for the hiring, termination or compensation of the other Party's employees or for any employee compensation or benefits of the other Party's employees. No employee or representative of a Party shall have any authority to bind or obligate the other Party for any sum or in any manner whatsoever, or to create or impose any contractual or other liability on the other Party without said other Party's approval. For all purposes, and notwithstanding any other provision of this Agreement to the contrary, the legal relationship under this Agreement of each Party to the other Party shall be that

of independent contractor. Nothing in this Agreement shall be construed to establish a relationship of partners or joint ventures between the Parties.

12.12 Performance by Affiliates. To the extent that this Agreement imposes obligations on Affiliates of a Party or permits a Party to exercise its rights or perform its obligations through its Affiliates, such Party agrees to cause its Affiliates to perform such obligations and shall guarantee performance of this Agreement by its Affiliates. If any disagreement arises out of the performance of this Agreement by an Affiliate of a Party, or the alleged failure of an Affiliate to comply with the conditions and obligations of this Agreement, the Party seeking to resolve such dispute shall have the right do so directly with the other Party, without any obligation to first pursue an action against, or recovery from, the Affiliate which is alleged to have caused a breach of this Agreement.

12.13 Construction. Each Party acknowledges that it has been advised by counsel during the course of negotiation of this Agreement, and, therefore, that this Agreement shall be interpreted without regard to any presumption or rule requiring construction against the Party causing this Agreement to be drafted. 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, schedule or exhibit, of or to, as the case may be, this Agreement. Except where the context otherwise requires, (a) wherever used, the use of any gender will be applicable to all genders; (b) the word "or" is used in the inclusive sense (and/or); (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 restriction on such amendments, supplements or modifications set forth herein or therein); (d) any reference to any Law refers to such Law 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; and (f) the words "include", "includes" and "including" shall be deemed to be followed by the phrase "but not limited to", "without limitation" or words of similar import.

[*Signature page follows*]

IN WITNESS WHEREOF, the Parties have executed this Collaboration and License Agreement as of the Effective Date.

UNIQURE BIOPHARMA B.V.	**4D MOLECULAR THERAPEUTICS, LLC**
BY: /s/ Jörn Aldag	BY: /s/ David Schaffer
NAME: Jörn Aldag	NAME: David Schaffer
TITLE: Chief Executive Officer	TITLE: Co-Founder, Member

Exhibit A

COMMITMENT LETTER FROM UNIQURE B.V.

Incorporated by reference to Exhibit 10.35 to the Company's Registration Statement on Form F-1 filed with the Commission on January 17, 2014

Schedule 1.5

4DMT PATENT RIGHTS

[**]

Schedule 1.41

OUTLINE OF BUDGET FOR RESEARCH PLAN

Confidential Materials omitted and filed separately with the Securities and Exchange Commission. A total of one page was omitted.

[**]

Schedule 1.54

DRAFT INVOICE

Invoice To:
<<Company name>>
<<address>>

Invoice No. **#**
Invoice Date: **<<date>>**

Attention: <<name, title>>
uniQure Contract No.:

Invoice From:
<<Company name>>
<<address>>

Description	Amount
Pursuant to <<contract and section reference>>	
<<payment type>>	$ 0.00
(i)	
(ii) Total Payment Due	**$ 0.00**
(iii)	

Wire Instructions:

Bank Name:
Bank Address:
Bank Contact:
Routing/transit:
Beneficiary:
Beneficiary Account #:

Payment Due: **<<contract payment terms>>**

Schedule 1.75

OUTLINE OF RESEARCH PLAN

Confidential Materials omitted and filed separately with the Securities and Exchange Commission. A total of one page was omitted.

[**].

OPTION AGREEMENT

This Option Agreement (the "Option Agreement") is made on the 17 of January 2014.

BY AND BETWEEN:

1 . Dr. David Kirn, with a business address at 19 Rima Court, Danville, CA 94526, 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 Participant will provide services for the benefit of the Company and its subsidiaries pursuant to a Collaboration and License Agreement by and between 4D Molecular Therapeutics, LLC ("4DMT") and uniQure Biopharma B.V. dated as of the date hereof (the "CLA").

- The Company wishes to grant to the Participant an Option as set out in this Option Agreement.

- The Company and the Participant intend that this Option Agreement and the Option comply with Section 409A (hereinafter referred to as "Section 409A") of the U.S. Internal Revenue Code of 1986, as amended (hereinafter referred to as the "Code").

THE PARTIES HERETO AGREE AS FOLLOWS:

Grant of Option

Article 1
The Company hereby grants to the Participant, solely subject to the approval of the shareholders of the Company holding a majority of the Company's Class A shares, an option (the "Option") to purchase up to 1,524,364 Class B Shares (the "Shares"). The Option is subject to the terms and conditions of this Option Agreement.

Option Period

Article 2
The Date of Grant of the Option is January 17, 2014.

Option Exercise Price

Article 3
The Option Exercise Price is €0.01 per Share.

Exercise; Cash Out

Article 4a
The Option can only be exercised if and to the extent the Option has vested, and has not expired or lapsed. Notwithstanding the foregoing, the Option shall vest on the dates set out below (each, a "Vesting Date") and be exercisable as follows:

- The Option shall vest as to 25% of the total number of shares subject to the Option (the "First Option Tranche") on October 1, 2014 (the "First Tranche Date"); provided that the CLA then remains in effect and has not been terminated in accordance with its terms and no notice of termination thereof has been given in accordance with Section 10.2(e) thereof. The First Option Tranche, solely to the extent such tranche vests in accordance with this Option Agreement, shall be exercisable by the Participant only between October 1, 2014 and December 28, 2014. If the First Option Tranche is not exercised by 5:00 pm Amsterdam Time on December 28, 2014, the First Option Tranche shall terminate as of such time and be of no further force or effect.

- The Option shall vest as to 50% of the total number of shares subject to the Option (the "Second Option Tranche") on January 31, 2015 (the "Second Tranche Date"); provided that the CLA then remains in effect and has not been terminated in accordance with its terms and no notice of termination thereof has been given in accordance with Section 10.2(e) thereof. The Second Option Tranche, solely to the extent such tranche vests in accordance with this Option Agreement, shall be exercisable by the Participant only between January 31, 2015 and December 28, 2015. If the Second Option Tranche is not exercised by 5:00 pm Amsterdam Time on December 28, 2015, the Second Option Tranche shall terminate as of such time and be of no further force or effect.

- The Option shall vest as to 25% of the total number of shares subject to the Option (the "Third Option Tranche", and each of the First Option Tranche, the Second Option Tranche and the Third Option Tranche shall be referred to as an

"Option Tranche"); on January 31, 2016 (the "Third Tranche Date"); provided that the CLA then remains in effect and has not been terminated in accordance with its terms and no notice of termination thereof has been given in accordance with Section 10.2(e) thereof. The Third Option Tranche,

solely to the extent such tranche vests in accordance with this Option Agreement, shall be exercisable by the Participant only between January 31, 2016 and December 28, 2016. If the Third Option Tranche is not exercised by 5:00 pm Amsterdam Time on December 28, 2016, the Third Option Tranche shall terminate as of such time and be of no further force or effect.

Neither the Participant nor the Company shall have the right to accelerate or defer the exercisability of the Option unless explicitly permitted or required by Section 409A.

Notwithstanding the foregoing, in the event the Company undergoes a "change in control event" within the meaning of Section 409A (a "409A Change in Control") prior to the Third Tranche Date, then (i) 50% of each Option Tranche that has not yet vested shall vest and become exercisable immediately prior to the closing of the 409A Change in Control, and (ii) the remaining 50% of each Option Tranche that has not yet vested shall vest in accordance with the provisions of Article 4a; and each case such vested Shares shall be exercisable only in accordance with Article 4a. With respect to any portion of an Option Tranche that becomes vested and exercisable in accordance with subclause (i) above, such portion of the Option Tranche shall be automatically exercised immediately prior to the Change in Control and for each Share subject to such portion, the Participant shall be entitled to receive the per Share merger consideration received by shareholders in connection with such 409A Change in Control, less the per Share exercise price; provided, however, that in the event that the consideration received in connection with such 409A Change in Control consists of anything other than cash and/or freely tradable securities, then, immediately following the closing of the 409A Change in Control, the Company shall pay to the Optionee an amount in cash equal to the product of (x) the number of Shares that become vested pursuant to subclause (i), multiplied by (y) the cash value of the per Share amount received by shareholders in connection with such 409A Change in Control less the per Share exercise price.

Article 4b
Notwithstanding anything to the contrary herein, in the event that, prior to January 31, 2015, the Company terminates the CLA pursuant to Section 10.2(c) (Termination for Futility) thereof, then (A) one-half of the number of Second Tranche Option Shares that would

otherwise have vested on January 31, 2015 will be deemed to vest and become exercisable on January 31, 2015 (notwithstanding the failure to satisfy any conditions to such vesting), and (B) the Option shall terminate with respect to one-half of the number of Second Tranche Option Shares that would otherwise have vested on January 31, 2015 and with respect to all of the Third Tranche Option Shares, and shall cease to be exercisable in respect of such Shares.

Article 4c
Notwithstanding anything to the contrary herein, but subject to Article 4b, in the event that the Company does not complete an underwritten public offering of its ordinary shares pursuant to an effective registration statement under the U.S. Securities Act of 1933 and the listing of its ordinary shares on the Nasdaq Global Market (an "IPO") prior to the First Tranche Date, the Second Tranche Date or the Third Tranche Date, as the case may be, the provisions of this Article 4c shall apply:

- If the Company has not completed an IPO by September 1, 2014, the Company may elect to pay to the Participant on the date 10 days prior to the First Tranche Date the Cash-Out Amount in respect of the First Option Tranche Shares.

- If the Company has not completed an IPO by December 31, 2014, the Company may further elect to pay to the Participant on the date 10 days prior to the Second Tranche Date the Cash-Out Amount in respect of the Second Option Tranche Shares.

- If the Company has not completed an IPO by December 31, 2015, the Company may further elect to pay to the Participant on the date 10 days prior to the Third Tranche Date the Cash-Out Amount in respect of the Third Option Tranche Shares.

"Cash-Out Amount" shall mean an amount in cash equal to the product of (x) the number of Shares that would otherwise have vested on the applicable Vesting Date, multiplied by (y) an amount per Share equal to the greater of €2.52 and the fair market value of each Share as of the applicable Vesting Date. For purposes hereof, such fair market value shall be determined in good faith by the Supervisory Board of the Company, provided that such fair market value shall be no lower than the then most-recent per Share amount paid in a third-party financing completed after the date hereof and prior to such applicable Vesting Date.

For the avoidance of doubt, the Option shall cease to be exercisable in respect of any Shares as to which the Company has elected to pay the relevant Cash-Out Amount, and shall terminate in full with respect to such Shares; provided, however, that this Option shall remain in full force and effect in accordance with its terms with respect to any further tranche of Shares.

Non transferability

Article 5
The Option granted to the Participant under this Option Agreement cannot 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 6.a.
Any wage or personal or corporate income tax, or social security premiums due in connection with the Option, 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 Option and the sale of the Shares derived from exercise of the Option, will be for the account of the Participant.

Article 6.b.
If the Option is not exercised, any tax and/or social security premiums paid will not be refunded or compensated for.

Adjustments

Article 7

Any adjustment made by the Company with respect to the Option shall be undertaken in a manner that complies with Section 409A. Without limiting the generality of the foregoing, the number and class of Shares subject to the Option and the exercise price per Share shall automatically be proportionately adjusted in the event of a share consolidation, share split, share reclassification or other similar event.

Section 409A

Article 8
The Option is intended to comply with the provisions of Section 409A and shall be construed and interpreted consistently therewith. Notwithstanding the foregoing, the Company shall have no liability to the Participant or any other person in the event that the Option is determined not to be compliant with Section 409A.

Governing law

Article 9.a.
The Option Agreement shall be governed by the laws of the Netherlands.

Article 9.b.
All disputes arising in connection with the Option Agreement shall be exclusively submitted to the jurisdiction of the competent court in Amsterdam, the Netherlands.

Acceptance

Article 10
The Participant hereby affirms his acceptance the Option granted to him including the conditions stipulated in this Option Agreement.

Duly signed in Amsterdam, on 17 January 2014

/s/ Jörn Aldag	/s/ David Kirn
uniQure B.V.	Participant
Name: Jörn Aldag, CEO	Name: Dr. David Kirn

OPTION AGREEMENT

This Option Agreement (the "Option Agreement") is made on the 17 of January 2014.

BY AND BETWEEN:

1 . Dr. David Schaffer, with a business address at 19 Rima Court, Danville, CA 94526, 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 Participant will provide services for the benefit of the Company and its subsidiaries pursuant to a Collaboration and License Agreement by and between 4D Molecular Therapeutics, LLC ("4DMT") and uniQure Biopharma B.V. dated as of the date hereof (the "CLA").
- The Company wishes to grant to the Participant an Option as set out in this Option Agreement.
- The Company and the Participant intend that this Option Agreement and the Option comply with Section 409A (hereinafter referred to as "Section 409A") of the U.S. Internal Revenue Code of 1986, as amended (hereinafter referred to as the "Code").

THE PARTIES HERETO AGREE AS FOLLOWS:

Grant of Option

Article 1
The Company hereby grants to the Participant, solely subject to the approval of the shareholders of the Company holding a majority of the Company's Class A shares, an option (the "Option") to purchase up to 1,524,364 Class B Shares (the "Shares"). The Option is subject to the terms and conditions of this Option Agreement.

Option Period

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The Date of Grant of the Option is January 17, 2014.

Option Exercise Price

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The Option Exercise Price is €0.01 per Share.

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The Option can only be exercised if and to the extent the Option has vested, and has not expired or lapsed. Notwithstanding the foregoing, the Option shall vest on the dates set out below (each, a "Vesting Date") and be exercisable as follows:

- The Option shall vest as to 25% of the total number of shares subject to the Option (the "First Option Tranche") on October 1, 2014 (the "First Tranche Date"); provided that the CLA then remains in effect and has not been terminated in accordance with its terms and no notice of termination thereof has been given in accordance with Section 10.2(e) thereof. The First Option Tranche, solely to the extent such tranche vests in accordance with this Option Agreement, shall be exercisable by the Participant only between October 1, 2014 and December 28, 2014. If the First Option Tranche is not exercised by 5:00 pm Amsterdam Time on December 28, 2014, the First Option Tranche shall terminate as of such time and be of no further force or effect.
- The Option shall vest as to 50% of the total number of shares subject to the Option (the "Second Option Tranche") on January 31, 2015 (the "Second Tranche Date"); provided that the CLA then remains in effect and has not been terminated in accordance with its terms and no notice of termination thereof has been given in accordance with Section 10.2(e) thereof. The Second Option Tranche, solely to the extent such tranche vests in accordance with this Option Agreement, shall be exercisable by the Participant only between January 31, 2015 and December 28, 2015. If the Second Option Tranche is not exercised by 5:00 pm Amsterdam Time on December 28, 2015, the Second Option Tranche shall terminate as of such time and be of no further force or effect.
- The Option shall vest as to 25% of the total number of shares subject to the Option (the "Third Option Tranche", and each of the First Option Tranche, the Second Option Tranche and the Third Option Tranche shall be referred to as an

"Option Tranche"); on January 31, 2016 (the "Third Tranche Date"); provided that the CLA then remains in effect and has not been terminated in accordance with its terms and no notice of termination thereof has been given in accordance with Section 10.2(e) thereof. The Third Option Tranche,

solely to the extent such tranche vests in accordance with this Option Agreement, shall be exercisable by the Participant only between January 31, 2016 and December 28, 2016. If the Third Option Tranche is not exercised by 5:00 pm Amsterdam Time on December 28, 2016, the Third Option Tranche shall terminate as of such time and be of no further force or effect.

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Notwithstanding anything to the contrary herein, but subject to Article 4b, in the event that the Company does not complete an underwritten public offering of its ordinary shares pursuant to an effective registration statement under the U.S. Securities Act of 1933 and the listing of its ordinary shares on the Nasdaq Global Market (an "IPO") prior to the First Tranche Date, the Second Tranche Date or the Third Tranche Date, as the case may be, the provisions of this Article 4c shall apply:

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- If the Company has not completed an IPO by December 31, 2015, the Company may further elect to pay to the Participant on the date 10 days prior to the Third Tranche Date the Cash-Out Amount in respect of the Third Option Tranche Shares.

"Cash-Out Amount" shall mean an amount in cash equal to the product of (x) the number of Shares that would otherwise have vested on the applicable Vesting Date, multiplied by (y) an amount per Share equal to the greater of €2.52 and the fair market value of each Share as of the applicable Vesting Date. For purposes hereof, such fair market value shall be determined in good faith by the Supervisory Board of the Company, provided that such fair market value shall be no lower than the then most-recent per Share amount paid in a third-party financing completed after the date hereof and prior to such applicable Vesting Date.

For the avoidance of doubt, the Option shall cease to be exercisable in respect of any Shares as to which the Company has elected to pay the relevant Cash-Out Amount, and shall terminate in full with respect to such Shares; provided, however, that this Option shall remain in full force and effect in accordance with its terms with respect to any further tranche of Shares.

Non transferability

Article 5
The Option granted to the Participant under this Option Agreement cannot 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 6.a.
Any wage or personal or corporate income tax, or social security premiums due in connection with the Option, 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 Option and the sale of the Shares derived from exercise of the Option, will be for the account of the Participant.

Article 6.b.
If the Option is not exercised, any tax and/or social security premiums paid will not be refunded or compensated for.

Adjustments

Article 7

Any adjustment made by the Company with respect to the Option shall be undertaken in a manner that complies with Section 409A. Without limiting the generality of the foregoing, the number and class of Shares subject to the Option and the exercise price per Share shall automatically be proportionately adjusted in the event of a share consolidation, share split, share reclassification or other similar event.

Section 409A

Article 8
The Option is intended to comply with the provisions of Section 409A and shall be construed and interpreted consistently therewith. Notwithstanding the foregoing, the Company shall have no liability to the Participant or any other person in the event that the Option is determined not to be compliant with Section 409A.

Governing law

Article 9.a.
The Option Agreement shall be governed by the laws of the Netherlands.

Article 9.b.
All disputes arising in connection with the Option Agreement shall be exclusively submitted to the jurisdiction of the competent court in Amsterdam, the Netherlands.

Acceptance

Article 10
The Participant hereby affirms his acceptance the Option granted to him including the conditions stipulated in this Option Agreement.

Duly signed in Amsterdam, on 17 January 2014

/s/ Jörn Aldag	/s/ David Schaffer
uniQure B.V.	Participant
Name: Jörn Aldag, CEO	Name: Dr. David Schaffer

Exhibit 10.35

UNIQURE B.V.

Meibergdreef 61
Amsterdam 1105 BA, The Netherlands

January 17, 2014

Dr. David Schaffer
Dr. David Kirn
4D Molecular Therapeutics, LLC
19 Rima Court
Danville, CA 94526

Re: Commitment Letter Pursuant to Collaboration and License Agreement

Gentlemen:

We refer to (1) the Collaboration and License Agreement by and between 4D Molecular Therapeutics, LLC ("4DMT") and uniQure Biopharma B.V. dated as of the date hereof (the "CLA"), and (2) the Grant Letters by and between uniQure B.V. and each of you dated as of the date hereof (the "Option Grants"). This letter is the Commitment Letter referred to in the Introduction to the CLA.

We hereby agree as follows:

1. Supervisory Board Membership. We hereby undertake to procure that Dr. Schaffer is appointed as a member of the Supervisory Board of uniQure B.V. (the "Supervisory Board") for a two-year term, commencing immediately following an extraordinary meeting of the shareholders of uniQure B.V. to be convened within 15 days of the date hereof(the "Initial Term"). At the expiration of such Initial Term, we agree that a person nominated by 4DMT shall be nominated by the Supervisory Board for election to a further three-year term as a member of the Supervisory Board, subject to approval by the shareholders of uniQure B.V. In the event that the CLA is terminated pursuant to Section 10.2 thereof prior to the expiration of the Initial Term or any subsequent term of office, Dr. Schaffer (or such other person as may be nominated by 4DMT from time to time) hereby agrees that he shall be deemed to have resigned his position as a member of the Supervisory Board effective immediately upon such termination of the CLA.

2. Supervisory Board Observer Rights. We hereby agree that Dr. Kirn shall have a right to attend all meetings of the Supervisory Board in a nonvoting observer capacity; provided, however, that Dr. Kirn agrees to hold in confidence all information provided; and provided further, that we reserve the right to withhold any information and to exclude Dr. Kirn from any meeting or portion thereof if access to such information or attendance at such meeting could adversely affect the attorney-client privilege between uniQure B.V. and its counsel or result in disclosure of trade secrets or a conflict of interest. The right provided by this paragraph 2 shall terminate on the first anniversary hereof.

3. Advisory Board Memberships. We hereby undertake to form a Scientific Advisory Board and a Clinical Advisory Board by no later than the first anniversary hereof. Upon the formation of each such committee, we undertake to appoint one nominee of 4DMT as a member of each such committee, such membership to continue during the Research Term (as defined in the CLA). The initial 4DMT nominee to the Scientific Advisory Board shall be Dr. Schaffer and the initial 4DMT nominee to the Clinical Advisory Board shall be Dr. Kirn.

The following sections of the CLA are hereby incorporated by reference herein and shall govern this letter: Article XI (Dispute Resolution); Section 12.1 (Governing Law); Section 12.2 (Waiver); Section 12.3 (Notices); Section 12.6 (Severability); Section 12.7 (Assignment); Section 12.8 (Counterparts); and Section 12.9 (Force Majeure).

*****

Very truly yours,

UNIQURE B.V.

By: /s/ Jörn Aldag
Name: Jörn Aldag
Title: Chief Executive Officer

ACKNOWLEDGED AND AGREED:

4D MOLECULAR THERAPEUTICS, LLC

By: /s/ Jörn Aldag
Name: Jörn Aldag
Title: Chief Executive Officer

/s/ David Schaffer

DR. DAVID SCHAFFER

/s/ David Kirn
DR. DAVID KIRN

Exhibit 23.1

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 17, 2014

/s/ drs. A.C.M. van der Linden RA

Exhibit 99.2

Consent of Director Nominee

uniQure B.V. has filed a Registration Statement on Form F-1 (Registration No. 333-193158) with the Securities and Exchange Commission under the Securities Act of 1933, as amended (the "Securities Act"), in connection with the initial public offering of ordinary shares of uniQure B.V. In connection therewith, I hereby consent, pursuant to Rule 438 of the Securities Act, to being named as a nominee to the supervisory board of uniQure B.V. in the Registration Statement, as may be amended from time to time. I also consent to the filing of this consent as an exhibit to such Registration Statement and any amendments thereto.

/s/ David Schaffer

Name: David Schaffer
Date: January 17, 2014