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## SECURITIES AND EXCHANGE COMMISSION

Washington D.C. 20549

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### FORM 20-F

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☐ REGISTRATION STATEMENT PURSUANT TO SECTION 12(b) OR (g) OF THE SECURITIES EXCHANGE ACT OF 1934

OR

☒ ANNUAL REPORT PURSUANT TO SECTION 13 OR 15(d) OF THE SECURITIES EXCHANGE ACT OF 1934

For the fiscal year ended December 31, 2014

OR

☐ TRANSITION REPORT PURSUANT TO SECTION 13 OR 15(d) OF THE SECURITIES EXCHANGE ACT OF 1934

For the transition period from \_\_\_\_\_ to \_\_\_\_\_

☐ SHELL COMPANY REPORT PURSUANT TO SECTION 13 OR 15(d) OF THE SECURITIES EXCHANGE ACT OF 1934

Date of event requiring this shell company report  
Commission file number: 001-36294

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### uniQure N.V.

(Exact name of Registrant as specified in its charter and translation of Registrant's name into English)

**The Netherlands**

(Jurisdiction of incorporation or organization)

**Meibergdreef 61, 1105BA Amsterdam, The Netherlands**  
(Address of principal executive offices)

**Jörn Aldag**  
**Chief Executive Officer**  
**Tel: +31 20 240-6000**

**Meibergdreef 61, 1105BA Amsterdam, The Netherlands**  
(Name, Telephone, E-mail and/or Facsimile number and Address of Company Contact Person)

Securities registered or to be registered pursuant to Section 12(b) of the Act:

<b>Title of each class</b>	<b>Name of each exchange on which registered</b>
Ordinary Shares	NASDAQ Global Select Market

Securities registered or to be registered pursuant to Section 12(g) of the Act: **None**

Securities for which there is a reporting obligation pursuant to Section 15(d) of the Act: **Ordinary Shares**

Indicate the number of outstanding shares of each of the issuer's classes of capital or common stock as of the close of the period covered by the annual report:

**18,092,194 Ordinary Shares**  
(as of December 31, 2014)

Indicate by check mark if the registrant is a well-known seasoned issuer, as defined in Rule 405 of the Securities Act.

☐ Yes ☒ No

If this report is an annual or transition report, indicate by check mark if the registrant is not required to file reports pursuant to Section 13 or 15(d) of the Securities Exchange Act of 1934.

☐ Yes ☒ No

Indicate by check mark whether the registrant (1) has filed all reports required to be filed by Section 13 or 15(d) of the Securities Exchange Act of 1934 during the preceding 12 months (or for such shorter period that the registrant was required to file such reports), and (2) has been subject to such filing requirements for the past 90 days.

☒ Yes ☐ No

Indicate by check mark whether the registrant has submitted electronically and posted on its corporate Web site, if any, every Interactive Data File required to be submitted and posted pursuant to Rule 405 of Regulation S-T (§232.405 of this chapter) during the preceding 12 months (or for such shorter period that the registrant was required to submit and post such files).

☒ Yes ☐ No

Indicate by check mark whether the registrant is a large accelerated filer, an accelerated filer, or a non-accelerated filer. See definition of "accelerated filer and large accelerated filer" in Rule 12b-2 of the Exchange Act. (Check one):

Large accelerated filer ☐ Accelerated filer ☒ Non-accelerated filer ☐

Indicate by check mark which basis of accounting the registrant has used to prepare the financial statements included in this filing:

U.S. GAAP ☐ International Financial Reporting Standards as issued by the International Accounting Standards Board ☒ Other ☐

If "Other" has been checked in response to the previous question, indicate by check mark which financial statement item the registrant has elected to follow:

☐ Item 17 ☐ Item 18

If this is an annual report, indicate by check mark whether the registrant is a shell company (as defined in Rule 12b-2 of the Exchange Act).

☐ Yes ☒ No

Indicate by check mark whether the registrant has filed all documents and reports required to be filed by Sections 12, 13 or 15(d) of the Securities Exchange Act of 1934 subsequent to the distribution of securities under a plan confirmed by a court.

☐ Yes ☐ No

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## General

As used herein, references to "we", "us", the "company", "uniQure", "uniQure B.V." or the "Group", or similar terms in this Form 20-F mean uniQure N.V. and, as the context requires, its subsidiaries. Effective February 10, 2014, we converted from a private company with limited liability (*besloten vennootschap met beperkte aansprakelijkheid*) to a public company with limited liability (*naamloze vennootschap*) under the laws of the Netherlands. In connection with this conversion, our legal name changed from uniQure B.V. to uniQure N.V.

Our financial statements are presented in Euros except where otherwise indicated, and are prepared in accordance with International Financial Reporting Standards ("IFRS") as issued by the International Accounting Standards Board ("IASB"). All references in this annual report to "Dollars" and "\$" are to US Dollars, and all references to "Euro" or "€" are to European Union Euro. For presentation purposes all financial information, including comparative figures from prior periods, have been stated in round thousands.

## Forward-Looking Statements

This annual report on Form 20-F contains forward-looking statements. The Private Securities Litigation Reform Act of 1995 provides a safe harbor from civil litigation for forward-looking statements accompanied by meaningful cautionary statements. Except for historical information, this report contains forward-looking statements within the meaning of Section 27A of the Securities Act of 1933, as amended, and Section 21E of the Securities Exchange Act of 1934, which may be identified by words such as "estimates", "anticipates", "projects", "plans", "seeks", "may", "will", "expects", "intends", "believes", "should" and similar expressions, or the negative versions thereof, and which also may be identified by their context. Such statements, whether expressed or implied, are based upon our current expectations and speak only as of the date made. We assume no obligation to update or revise any forward-looking statements even if experience or future changes make it clear that any projected results expressed or implied therein will not be realized.

These statements are subject to various risks, uncertainties and assumptions. Our actual results of operations may differ materially from those stated in or implied by such forward-looking statements as a result of a variety of factors, including those described under "Risk Factors" and elsewhere in this annual report.

**PART I**

**ITEM 1: IDENTITY OF DIRECTORS, SENIOR MANAGEMENT AND ADVISERS**

Not applicable.

**ITEM 2: OFFER STATISTICS AND EXPECTED TIMETABLE**

Not applicable.

**ITEM 3: KEY INFORMATION**

**A. Selected Financial Data**

The selected consolidated financial data as of December 31, 2013 and 2014 and for each of the years ended December 31, 2012, 2013 and 2014 have been derived from our audited consolidated financial statements and notes thereto set forth in Item 18 of this annual report. The selected consolidated financial data for the year ended December 31, 2010, 2011 and 2012 are derived from the audited consolidated financial statements not appearing in this annual report.

The following selected consolidated financial data should be read in conjunction with our "Operating and Financial Review and Prospects" and our consolidated financial statements and related notes appearing elsewhere in this annual report. Our financial statements are prepared in accordance with IFRS.

Note 1 to the financial statements contains additional information relating to the business combination between uniQure and Amsterdam Molecular Therapeutics, and the presentation of a continuous trading history.

**Consolidated Statements of Comprehensive Income Data:**

€ in thousands (except share and per share data)	YEARS ENDED DECEMBER 31,			
	2011	2012	2013	2014
License revenues	—	—	440	883
Collaboration revenues	—	—	2,503	3,802
Total revenues	—	—	2,943	4,685
Cost of goods sold	—	—	(800)	0
Other income	2,192	649	585	773
Research and development expenses	(15,500)	(10,231)	(13,182)	(33,932)
Selling, general and administrative expenses	(3,807)	(4,564)	(11,628)	(11,167)
Other gains / (losses)—net	(26)	(45)	(453)	5,807
Total Operating Costs	(19,333)	(14,840)	(25,263)	(39,292)
Operating result	(17,141)	(14,191)	(22,535)	(33,834)
Finance income	277	22	102	254
Finance expense	(436)	(547)	(4,387)	(3,460)
Finance income/(expense)—net	(159)	(525)	(4,285)	(3,206)
Result before corporate income taxes	(17,300)	(14,716)	(26,820)	(37,040)
Corporate income taxes	—	—	0	0
Net Loss	(17,300)	(14,716)	(26,820)	(37,040)
Items that may be subsequently reclassified to profit or loss	—	—	12	1,149
Other comprehensive income	—	—	12	1,149
Total comprehensive loss	(17,300)	(14,716)	(26,808)	(35,891)
Loss per share attributable to the equity holders of the company during the year				
Basic and diluted loss per share	(3.65)	(1.70)	(2.48)	(2.16)

The total number of ordinary shares outstanding at December 31, 2011, 2012, 2013 and 2014 was 4,749,625, 9,653,495, 12,194,906 and 18,092,194, respectively. The share capital at December 31, 2011, 2012, 2013 and 2014 was €237,000, €483,000, €610,000 and €905,000 respectively.

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	2013	2014
Cash and cash equivalents	17,859	1,100	263	23,810	53,219
Total assets	22,703	5,804	5,567	38,969	95,786
Total debt	4,621	4,544	1,498	7,864	17,270
Accumulated deficit	(88,205)	(105,505)	(117,234)	(144,041)	(181,081)
Total shareholders' equity (deficit)	13,659	(2,593)	(448)	5,564	43,084

**Exchange Rate Information**

Our business is primarily conducted in the European Union, and we maintain our books and records in Euro. We have presented results of operations in Euro. In this annual report, translations from Euro to US dollars were made at a rate of €0.8237 to \$1.00, the official exchange rate quoted by

the European Central Bank at the close of business on December 31, 2014. As of March 31, 2015, the official exchange rate of Euro to US dollars was €0.928 to \$1.00. Such US dollar amounts are not necessarily indicative of the actual amounts of US dollars which could have been actually purchased on exchange of Euro on the dates indicated.

	<u>Period-end</u>	<u>Average for period</u> (€ per U.S. dollar)	<u>Low</u>	<u>High</u>
<b>Year Ended December 31,</b>				
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	0.724	0.725	0.721	0.729
2015 (to April 1)	0.928	0.698	0.826	0.925

**B. Capitalization and Indebtedness**

Not applicable.

**C. Reasons for the Offer and Use of Proceeds**

Not applicable.

## D. Risk Factors

### **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 had a net loss of €37.0 million in 2014, €26.8 million in 2013 and €14.7 million in 2012. As of December 31, 2014, we had an accumulated deficit of €181.1 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. Although our recently signed agreement with Bristol-Myers Squibb Company, or BMS, will, following closing, provide us with substantial near-term collaboration and equity financing, a significant portion of the potential consideration is contingent on achieving research, development, regulatory and sales milestones. We expect to continue to incur significant expenses and losses over the next several years, and our net losses may fluctuate significantly from quarter to quarter and year to year. We anticipate that our expenses will increase substantially as we:

- conduct our Phase I/II clinical trial of AMT-060 for hemophilia B in collaboration with Chiesi;
- complete our EMA-mandated post-approval clinical trials of Glybera;
- conduct a clinical trial of Glybera, either as part of the EMA-mandated post-approval clinical trials or separately, to obtain data needed to file a BLA for Glybera with the FDA, and seek marketing approval for Glybera in the United States and other countries;
- 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 validation of and commence commercial manufacturing at our facility in Lexington, Massachusetts;
- 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 groups;
- continue to add operational, financial and management information systems and related compliance personnel; and
- continue to operate as a public company.

We and our collaborators may never succeed in these activities and, even if we do, may never generate revenues that are sufficient to achieve or sustain 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.



***We will likely need to raise additional funding, which may not be available on acceptable terms, or at all. Failure to obtain capital when needed may force us to delay, limit or terminate our product development efforts or other operations which could have a material adverse effect on our business, financial conditions, results of operations and cash flows.***

We expect to incur significant expenses in connection with our ongoing activities. Although our recently signed agreement with BMS will, following closing, provide us with substantial near-term collaboration and equity financing, a significant portion of the potential consideration is contingent on achieving research, development, regulatory and sales milestones. We expect that we will likely need to obtain substantial additional funding in connection with our continuing operations. In addition, 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.

Adequate capital may not be available to us when needed or may not be available on acceptable terms. 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. If we raise additional capital through the sale of equity or convertible debt securities, our shareholders' ownership interest will be diluted, and the terms of these securities may include liquidation or other preferences that adversely affect the rights of holders of our ordinary shares. If we raise additional funds through collaborations, strategic alliances, or marketing, distribution or licensing arrangements with third parties, we may have to issue additional equity, 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 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, results of operations and cash flows. See also "—Risks Related to Our Dependence on Third Parties—If our collaboration with BMS does not close or is not successful, our development plans, financial position and opportunities for growth may be adversely affected."

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

As of December 31, 2014, we had €16.4 million of outstanding borrowings under our Loan and Security Agreement with Hercules, which we are required to repay in monthly principal installments from January 2016 through June 2018. 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, research and 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 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.

***Our revenues will depend in part on the commercial success of sales of Glybera.***

We anticipate that the first patient will be treated with Glybera in the European Union in mid-2015, although such revenues are initially likely to be modest. 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 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;
- we may be unable to manufacture Glybera to the quality specifications required in a required time frame or in quantities necessary to timely satisfy demand for Glybera;
- we may be unable to maintain our marketing approval for Glybera in the European Union if it is determined by the EMA that there are safety, quality, efficacy or other material concerns associated with Glybera; and
- coverage, pricing and reimbursement levels may be lower than we expect.

If we fail to achieve anticipated revenues from this product, it may have an adverse effect on our results of operations and cause the value of our ordinary shares to decline.

***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 number 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.

### **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 product pipeline and to progress these candidates through clinical development ourselves or 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. 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, genes 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 competitors may be unwilling to license rights to us. Furthermore, we may be unable to identify suitable product candidates or technologies within our areas 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 and impediments to the progress of 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, but are not limited to:

- 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;
- 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 or otherwise properly manage the clinical trial process, including the retention of proper case files;
- 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.

Before obtaining marketing approval from regulatory authorities for the sale of our product candidates, we must conduct extensive clinical trials to demonstrate the safety and efficacy of the product candidates in humans. It is impossible to predict when or if any of our clinical trials will demonstrate that product candidates are effective or safe in humans. 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.

Because of the nature of the gene therapies we are developing, regulators may also require us to demonstrate long-term gene expression or clinical efficacy, which may require longer clinical trial

periods or longer patient follow-up than we currently expect or is typically required in the case of other therapies.

Our ability to recruit patients for our trials is often reliant on third parties, such as the pharmacies at our clinical trial sites. These third parties may not have the adequate infrastructure established to handle gene therapy products to support certain gene therapy product formulations, in order for them to agree to recruit patients on our behalf. To the extent that the infrastructure cannot be established at the pharmacies we may experience delays in recruiting patients for our studies. For example, we deliver clinical supplies for our hemophilia B trial in vials, which must be combined into infusion bags, and we have been informed that certain pharmacies at some prospective clinical trial sites are not able to undertake this procedure.

In addition, 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. This may result in our failure to initiate or continue clinical trials for our product candidates, or may cause us to abandon one or more clinical trials altogether. In particular, because several of our programs are focused on the treatment of patients with orphan or ultra-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.

Any inability to successfully initiate or complete preclinical and clinical development could result in additional costs to us or impair our ability to receive marketing approval, to generate revenues from product sales, or obtain 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.

***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 serotypes, 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.

***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. For example, 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. In addition, two gene therapy studies in 2003 were terminated after five subjects developed leukemia.

Although none of our current product candidates utilize the 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.

Glybera or our product candidates may prove to have undesirable or unintended side effects, toxicities or other characteristics that may require us to abandon or limit their development, preclude our obtaining additional marketing approval or prevent or limit commercial use. In our clinical trials for Glybera, there were, as at March 20, 2015, a total of 55 serious adverse event reports in Glybera-treated patients, two of which were assessed as potentially related to Glybera, one incidence of pulmonary embolism and one incidence of fever. In our partner's clinical development program for AIP, there were four serious adverse events, none of which was determined by the investigator to be treatment-related.

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, failure of the medical community to accept and prescribe gene therapy treatments, potential regulatory delays in the testing or approval of our 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 Regulatory Approval**

***We cannot predict when or if we will obtain marketing approval to commercialize a product candidate or, in the case of Glybera, further marketing approval in jurisdictions outside the European Union, and any approval we receive may be for a more narrow indication than we expect.***

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 FDA and other regulatory agencies in the United States, the EMA and other regulatory agencies of the member states of the European Union, and similar regulatory authorities in other jurisdictions. 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. 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. 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. 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 and December 2014, 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 submitted a protocol for this trial to the FDA. We are seeking to amend the protocol for our EU post-approval trial of Glybera so that it could also serve as a clinical program with a design that addresses the FDA's requirements and are in preliminary discussions with EU regulatory authorities regarding the acceptability of the amended protocol. We intend to file a special protocol assessment with the FDA in first half of 2015 in respect of this trial design. 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 in 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. 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. 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 marketing approval or if we fail to maintain approval of Glybera in the European Union or to 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 the relevant committees had initially reached negative decisions on 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.

Following our further submissions, 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. If these post-approval studies do not produce data that support the results of our original development program for Glybera, the marketing authorization for Glybera in the European Union could be withdrawn.

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.

***The FDA will require us to conduct comparability studies evaluating the products manufactured at our Amsterdam facility with those to be manufactured at our new Lexington, Massachusetts facility. 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, or begin manufacturing at a new facility, 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 or facility. In connection with any application for marketing approval 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 new facility in Lexington, Massachusetts. 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. 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 potential 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 obligations, review and other regulatory requirements for Glybera in the European Union, and any of our product candidates for which we obtain marketing approval in the future could be subject to similar requirements, which may restrict or eliminate the commercial success of Glybera or our other product candidates.***

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.

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 activities. Specifically, as a condition to approval of Glybera 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, implement an additional manufacturing process step, comply with certain notification obligations and undergo annual reassessment, any negative outcome of which could potentially lead to a withdrawal of marketing approval for Glybera. 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, particularly in light of the relatively small market for this orphan indication. Non-compliance with EU requirements regarding safety monitoring or pharmacovigilance can also result in significant financial penalties.

In addition, we have submitted several Type II variations to the EMA, which seek to update the summary product of characteristics, SmPC, of Glybera to include additional six-year follow-up and other clinical data. Following our submission of the Type II variations, and a voluntary disclosure of accidental destruction of some historical source data at a site in Canada, the EMA requested a good clinical practices, or GCP, inspection of our Glybera trial program. The Dutch and UK regulatory authorities conducted the inspection on behalf of the EMA in early 2015. The inspectors reported to the EMA on the quality control mechanisms that were in place in our company during data acquisition and processing in 2009 and 2010 with respect to maintaining patient and trial data obtained prior to approval of Glybera, and the integrity of the historical trial data as a whole. We have already implemented corrective quality control actions to rectify the oversight issues identified by the inspection and continue to refine our quality system. We believe that the events in the past do not materially affect the previously reported results of our historical trials. The inspection team also concluded "that the quality of the data and the level of GCP compliance both are acceptable" and that the trial data can be used for the submitted Type II variations.

As part of the ongoing variation procedure, the EMA is currently reviewing the risk/benefit analysis of Glybera in light of the additional six-year follow-up data we provided and the findings of the GCP inspection. Although we believe that these data support the conclusions that led to the original approval of Glybera under exceptional circumstances, there can be no assurance that the EMA or its committees will not reach a different conclusion. We anticipate that the CAT and CHMP will review this matter during its meetings in April 2015, and expect to receive a response setting out the EMA's preliminary position around April 23, 2015. We anticipate this response will include requests for additional information as part of the variation procedure, which may require a further response from us to support our position. Any adverse outcome of this review could require us to expend significant further resources to support our conclusions, including potentially conducting further post-approval

studies, or could potentially result in revocation of the marketing approval for Glybera in the European Union.

Should we receive FDA approval of Glybera or any of our other product candidates in the future, 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, also 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.

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 or may lead to withdrawal of marketing approval, which would have a material adverse effect on our business.

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

We believe that all of our current product candidates, including Glybera, will be viewed as gene therapy products by the applicable regulatory authorities. Gene therapies are relatively new treatments for which regulators do not have extensive experience or standard review and approval processes. 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.

Both the FDA and EMA 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, 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. 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. The close regulatory scrutiny of gene therapy products may result in delays and increased costs, and may ultimately lead to the failure to obtain approval for any gene therapy product.

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 EU legislation that governs advanced therapy medicinal products, including gene therapy products, which could result in changes in the data we need to submit to the EMA in order for our product candidates to gain regulatory approval or change the requirements for tracking, handling and distribution of the products which may be associated with increased costs. In addition, divergent scientific opinions among the various bodies involved in the review process may result in delays, require additional resources and ultimately result in rejection. For further discussion about the regulation we face in Europe and the United States, please see "Information on the Company—Business Overview—Government Regulation and Reimbursement."

Agencies at both the U.S. federal and state level, as well as congressional committees and foreign governments, have sometimes expressed interest in further regulating biotechnology. 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. 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.

These regulatory agencies, committees and advisory groups and the new regulations and 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 our product candidates or lead to significant post-approval limitations or restrictions. 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.

***Our failure to obtain or maintain orphan product exclusivity for any of our product candidates for which we seek this status could limit our commercial opportunity, and 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. Generally, if a product with an orphan drug designation subsequently receives the first marketing approval for the relevant indication, 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 EMA, however, may subsequently approve a similar drug for the same indication during the first product's market exclusivity if the EMA 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. 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.

We have obtained orphan designation for Glybera in the European Union and the United States. If we lose orphan drug exclusivity for Glybera or if our competitors obtain orphan drug exclusivity in indications related to our other product candidates before we do, we may lose out on the potential benefits of market exclusivity or be precluded from obtaining marketing authorization for our product candidate.

### **Risks Related to Commercialization**

***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 on the successful development and eventual commercialization of our product candidates and, in the mid-term, the successful commercialization of Glybera. The success of our product candidates will depend on a number of factors, including:

- successful completion of preclinical studies and clinical trials;
- receipt and maintenance of marketing approvals from applicable regulatory authorities;
- our ability to timely manufacture sufficient quantities according to required quality specifications;

- obtaining and maintaining patent and trade secret protection and non-patent, orphan drug exclusivity for our product candidates;
- obtaining and maintaining regulatory approval for our 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 resellers on acceptable terms in jurisdictions where we plan to utilize third parties for the marketing and sale of Glybera or other product candidates;
- 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 our gene therapies, including Glybera, may be smaller than we or third parties currently project, which may affect the size of our addressable markets.***

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 our therapies, are estimates based on our knowledge and understanding of these diseases. The total addressable market opportunities for these therapies 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 consent, 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 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 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, any of which would adversely affect our results of operations and our business.

***Any approved gene therapy we seek to offer 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. 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 compared with 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 outcomes. 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.

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

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 or maintaining 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. For example, in the second half of 2014 we encountered problems with the consistency and stability of the manufacturing process for Glybera. We have developed an improved manufacturing process for Glybera, which addresses also our post-approval commitment, and will conduct consistency and comparability studies in respect of this process, which we expect to submit to the EMA in mid-2015. Although these manufacturing issues are specific to Glybera and do not affect the manufacturing of our other product candidates, any failure to introduce and receive approval for our improved Glybera manufacturing process on schedule could adversely affect our ability to meet our obligations under our agreement with Chiesi, which could result in modest financial penalties and potential reputational harm.

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, Avalanche, Baxter, BioMarin, bluebird bio, Celladon, Dimension/Regen X, Isis, Oxford BioSciences, Sangamo BioScience, Spark Therapeutics and Voyager, 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 Amgen, Baxter, Bayer, Biogen Idec, BioMarin, Genzyme, Novartis, Novo Nordisk, Pfizer and Shire. 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.

## Risks Related to Our Dependence on Third Parties

***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 BMS for the development and commercialization of gene therapies for cardiovascular and potentially other diseases, with Chiesi, for both co-development and commercialization of our hemophilia B program and commercialization of Glybera in the European Union and certain other countries, and development programs with Institut Pasteur and UCSF for our development programs in Sanfilippo B syndrome and Parkinson's disease, respectively.

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;
- we generally have limited or no control over the design or conduct of clinical trials sponsored by our current 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 develop, independently or 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 achieves regulatory approval may not commit sufficient resources to the marketing and distribution of such product or products;

- disagreements with collaborators, including 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 rights 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 future research funding or milestone or royalty payments under the collaboration, and we may lose access to important technologies and capabilities of the collaboration. All of the risks relating to product development, regulatory approval and commercialization described herein also apply to the activities of our development collaborators.

***If our collaboration with BMS does not close or is not successful, our development plans, financial position and opportunities for growth may be adversely affected.***

Our recently signed collaboration with BMS for the development and commercialization of gene therapies for cardiovascular and potentially other diseases is subject to customary closing conditions, including clearance under the Hart-Scott-Rodino Antitrust Improvements Act. We also must obtain shareholder approval of our proposed equity issuances to BMS. If HSR clearance is not received, we would not be able to close this collaboration. In addition, if our shareholders do not approve the issuance of all shares issuable under our agreements with BMS, we would issue to BMS a lower number of our ordinary shares and would therefore receive lower proceeds from these equity arrangements than anticipated, which may limit our ability to advance our other development programs in the manner we intend.

In order to earn all milestone payments and royalties potentially due under this collaboration, we are dependent on BMS electing to designate and actively pursue target indications covered by the collaboration and we achieve meet all development, clinical and regulatory milestones under the collaboration. If BMS designates or actively pursues fewer development targets, or if we fail to achieve a significant number of the applicable milestones, the total payments we receive under this collaboration will be materially lower than are potentially payable. See also "Item 3—Key Information—Collaborations—Bristol-Myers Squibb Collaboration."

***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. Collaborations also allow us to share the costs of larger development and commercialization efforts with partners with greater resources. 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 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.

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.

We do not currently have a sales or marketing infrastructure and have no experience in the sale, marketing or distribution of pharmaceutical products. 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 territories outside the European Union and certain other countries, or we 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 of Glybera and of our hemophilia B and other product candidates in our new facility 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. 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. For example, in the second half of 2014 we encountered problems with the consistency and stability of the manufacturing process for Glybera. We have developed an improved manufacturing process for Glybera, which addresses also our post-approval commitment, and will conduct consistency and comparability studies in respect of this process, which we expect to submit to the EMA in mid-2015. Although these manufacturing issues are specific to Glybera and do not affect the manufacturing of our other product candidates, any failure to introduce and receive approval for our improved Glybera manufacturing process on schedule could adversely affect our ability to meet our obligations under our agreement with Chiesi, which could result in modest financial penalties and potential reputational harm.

A number of factors common to the manufacturing of most biologics and drugs could also cause production interruptions, including equipment malfunctions, facility contamination, labor problems, raw

materials shortages or contamination, natural disasters, disruption in utility services, terrorist activities, human error or disruptions in the operations of our suppliers. We also may encounter problems in hiring and retaining the experienced specialist personnel needed to operate 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 processes 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, result in delays in our clinical development or marketing schedules and harm our business.

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

Our manufacturing facility in Lexington, Massachusetts of approximately 53,000 square feet, will require regulatory approval. If regulatory approval 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 facilities are subject to significant government regulations and approvals. If we fail to comply with these regulations or maintain these approvals our business will be materially harmed.***

Our manufacturing facilities in Amsterdam and Lexington are 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 sale 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.

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; requiring us 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; requiring us to suspend 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 are subject to national, federal, state and local laws and regulations in the United States and 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 rely on licenses of intellectual property from third parties, and such licenses may not provide adequate rights or may not be available in the future on commercially reasonable terms or at all, and 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 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 genes and 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, or if the scope of the patent protection is not sufficiently broad, 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. Successful challenges to our patents 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.

The patent prosecution process is expensive, time-consuming and uncertain, 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. Additionally, 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.

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, EU patent law with respect to the patentability of methods of

treatment of the human body is more limited than U.S. 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.

***We may become involved in lawsuits to protect or enforce our patents or other intellectual property or third parties may assert their intellectual property rights against us, 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. An adverse result in any litigation proceeding could put one or more of our patents at risk of being invalidated, maintained in more narrowly amended form 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, increase our operating losses, reduce available resources 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, which could have an adverse effect on the price of our ordinary shares.

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. 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. We may not be able to obtain the 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 or otherwise to cease using the relevant intellectual property. In addition, we could be found liable for monetary damages, including treble damages and attorneys' fees if we are found to have willfully infringed a patent. A finding of infringement could prevent us from commercializing our product candidates or force us to cease or materially modify 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. Additionally, in the United States and some foreign jurisdictions, legislative and regulatory changes regarding the healthcare system and insurance coverage could result in more rigorous coverage criteria and downward pressure on drug prices, and may affect our ability to profitably sell any products 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 are considered not cost-effective or where a 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 a particular jurisdiction. 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 our therapies 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 and ultra-orphan diseases are relatively small. If we are unable to obtain adequate levels of reimbursement relative to the small market size in our target orphan and ultra-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 re-administer 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.

### **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. Industry associations also closely monitor the activities of member companies. 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, the federal False Claims Act, the federal Health Insurance Portability and Accountability Act of 1996, federal law that requires applicable manufacturers of covered drugs to report payments and other transfers of value to physicians and teaching hospitals and certain state and local laws applicable to pharmaceutical companies. We are also subject to the U.S. Foreign Corrupt Practices Act.

Efforts to ensure that our business arrangements with third parties will comply with applicable laws and regulations will involve substantial costs. 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 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 €9,500,000 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. 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 Chief Scientific Officer, Harald Petry, as well as the other principal members of our management, scientific and clinical team. Although we have entered into employment agreements with our senior management, each of them may terminate their employment on relatively short notice. We do not maintain "key person" insurance for any of our senior management or employees.

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.

If we are unable to continue to attract and retain high quality personnel, our ability to pursue our growth strategy will be limited.

***We are expanding our key capabilities and, as a result, may encounter difficulties in managing our growth, which could disrupt our operations.***

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 have experienced and expect in the future to continue 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 current and 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 additional sales force, marketing, manufacturing or other personnel 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 such personnel. 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 or other countries in which we may receive marketing approval.

#### **Risks Related to Our Ordinary Shares**

***The price of our ordinary shares has been and may in the future be volatile and fluctuate substantially.***

Our share price has been and may in the future be volatile. From the start of trading of our ordinary shares on the NASDAQ Global Select Market on February 4, 2013 through March 31, 2015, the sale price of our ordinary shares ranged from a high of \$28.00 to a low of \$8.29. The closing price on April 6, 2015 was \$33.61 per ordinary share. 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. 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.

***An active trading market for our ordinary shares may not be sustained.***

Although our ordinary shares are listed on The NASDAQ Global Select Market, an active trading market for our shares may not be sustained. If an active market for our ordinary shares does not continue, it may be difficult for our shareholders to sell their shares without depressing the market price for the shares or sell their shares at all. Any inactive trading market for our ordinary shares may also impair our ability to raise capital to continue to fund our operations by selling shares and may impair our ability to acquire other companies or technologies by using our shares as consideration. 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.

***Our senior managers, directors and major shareholders, if they choose to act together, will continue to have a significant degree of control with respect to matters submitted to shareholders for approval.***

Our management board and supervisory board members, senior management, and our shareholders and their affiliates who own more than 5% of our outstanding ordinary shares, in the aggregate, beneficially own approximately 63.7% 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, shareholders 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 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:

- 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 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 are a foreign private issuer and, as a result, we are not subject to U.S. proxy rules and are 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.***

We 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, our shareholders may not have the same protections afforded to shareholders of companies that are not foreign private issuers. 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 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.***

If we lose foreign private issuer 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 any 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 will continue to incur significant costs as a result of operating as a public company, and our management will be required to continue 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 \$1.5 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 Select 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 our initial public offering in February 2014, we were a private company with limited accounting personnel and other resources with which to address our internal controls and procedures. In connection with the preparation and external audit of our consolidated financial statements as of and for the year ended December 31, 2014 and our management's assessment of our internal control over financial reporting, we and our auditors, an independent registered public accounting firm, noted three material weaknesses in our internal control over financial reporting. We and our independent registered public accounting firm had identified the same three material weaknesses in our internal control over financial reporting in connection with the audit of our consolidated financial statements as of and for year ended December 31, 2013. Our independent registered public accounting firm has not conducted an audit of our internal control over financial reporting. 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.

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 control deficiency, or combination of control deficiencies, in internal control over financial reporting, such that there is a reasonable possibility that a material misstatement of our annual or interim financial statement will not be prevented or detected. In response, we are implementing several remedial actions to address these material weaknesses though we cannot guarantee when these material weaknesses will be fully remediated. For details, see "Item 15—Controls and Procedures."

If we fail to achieve and maintain the adequacy of our internal control over financial reporting, as the applicable 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. 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 Select Market, regulatory investigations and civil or criminal sanctions. We may also be required to restate our financial statements for prior periods. Our reporting and compliance 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 the required remediation efforts.

***We 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 the rights of our shareholders will differ from the rights of shareholders of a domestic U.S. issuer.***

As a foreign private issuer whose ordinary shares are listed on The NASDAQ Global Select 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 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 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.***

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 Select 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. Because we do not comply with all the provisions of the DCGC, shareholders 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 may 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, the expected price of our shares, 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 2014 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, 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 "Additional Information—Taxation—Taxation in the United States—Passive foreign investment company considerations."

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

We are incorporated under the laws of 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 shareholders 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.



Therefore U.S. shareholders 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—Comparison of Dutch corporate law and our Articles of Association and Delaware corporate law" in our F-3 registration statement.

## **ITEM 4: INFORMATION ON THE COMPANY**

### **A. History and Development of the Company**

uniQure was incorporated on January 9, 2012 as a private company with limited liability (*besloten vennootschap met beperkte aansprakelijkheid*) under the laws of the Netherlands. Our business was founded in 1998 and was initially operated through our predecessor company, Amsterdam Molecular Therapeutics (AMT) Holding N.V. or AMT. In 2011, AMT undertook a corporate reorganization, pursuant to which uniQure B.V. acquired the entire business and assets of AMT and completed a share-for-share exchange with the shareholders of AMT in the first half of 2012. Effective February 10, 2014, in connection with our initial public offering and pursuant to a deed of amendment and conversion, we converted into a public company with limited liability (*naamloze vennootschap*). Our legal name changed from uniQure B.V. to uniQure N.V. at the time of the conversion.

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 located at Meibergdreef 61, Amsterdam 1105 BA, the Netherlands, and our telephone number is +31 20 240 6000. Our website address is [www.uniquire.com](http://www.uniquire.com). Information on our website is not incorporated by reference into this annual report or any other report we file with or furnish to the SEC. Our ordinary shares are traded on the NASDAQ Global Select Market under the symbol "QURE".

### **B. Business Overview**

We are a leader in the field of gene therapy and have a technology platform that we use as the basis for our proprietary and collaborative product lines across multiple therapeutic areas. Our core gene therapies include AMT-060 for the treatment of hemophilia B, in which we initiated a Phase I/II clinical trial in the first quarter of 2015; our preclinical S100A1 therapeutic for the treatment of congestive heart failure and Glybera, the first and currently the only gene therapy product to receive regulatory approval in the European Union.

Our aim is to make gene therapy a mainstay of modern medicine by:

- using our technology platform to develop our own programs in liver-based diseases, cardio/metabolic diseases, and central nervous system, or CNS, diseases. Our focus is on areas in which we believe the modular nature of our approach offers the potential to reduce development risk, cost and time to market by allowing us to advance multiple programs using validated components of our technology and relying on safety and efficacy data from earlier clinical studies;
- sponsoring and acquiring additional early-stage programs in these areas from other biopharmaceutical companies and academic investigators;
- enhancing and accelerating these programs through our modularized research and development platform and our experience in the EU and FDA regulatory environments for gene therapies;
- applying our proprietary, commercial-scale manufacturing process to produce high quality material for our own and our collaborators' programs; and
- collaborating with pharmaceutical companies with the necessary expertise to enhance our late-stage therapy development and maximize the value of our therapies at the commercialization stage.

We believe that our technology platform and strategic collaborations place us at the forefront of gene therapy within our chosen therapeutic areas. Our transgene delivery system is based on common, adeno-associated viruses, or AAV, which we believe are safe and effective delivery methods for efficient expression of transgenes. We have the exclusive or non-exclusive rights to natural AAV serotypes for lipoprotein lipase deficiency, or LPLD, liver and CNS applications and the capability to identify and develop synthetic AAV vectors that are designed to optimize the expression of a particular transgene in specific tissue types. We produce our AAV-based vectors in our own facilities with a proprietary, commercial-scale, consistent, manufacturing process using insect cells and baculoviruses, a common family of viruses found in invertebrates. We believe our Lexington, Massachusetts-based facility, which is currently being qualified, is one of the world's leading, most versatile, gene therapy manufacturing facilities. We believe this technology platform, combined with our know-how derived from achieving the first regulatory approval of a gene therapy in the European Union, provides us a significant advantage in bringing our gene therapy products to the market ahead of our competitors.

We seek to develop gene therapies targeting a range of liver-based, cardio/metabolic and CNS indications, from ultra-orphan diseases, such as LPLD (for which Glybera is designated), to orphan diseases such as hemophilia B and Sanfilippo B syndrome, to common diseases that affect far larger populations, such as congestive heart failure and Parkinson's disease. The core of our approach is our modular technology backbone, which allows us to advance our programs in multiple therapeutic areas using validated components of our technology and safety and efficacy data from earlier clinical studies, in multiple therapeutic areas, with the potential to reduce development risk, cost and time to market. As part of our strategy, we are accessing important medical expertise for our therapeutic focuses through strong ties with academic thought leaders and clinical institutions. For cardio/metabolic diseases we are building a center of expertise in our German subsidiary, uniQure GmbH, in close cooperation with leading academic clinicians and surgeons at the university hospital and heart center in Heidelberg, Germany. Our CNS activities are based on collaborations with the University of California at San Francisco, the National Institutes of Health, and the Institut Pasteur, Paris, France. Our hemophilia B product originates from St. Jude Children's research Hospital in Memphis, Tennessee. We also seek to collaborate with or acquire emerging companies within our chosen therapeutic areas that are conducting or sponsoring early-stage clinical trials. Our collaborations allow us to cost-effectively obtain access to pre-clinical and early-stage programs without expending significant resources of our own. We generally have the rights to the data generated in these collaborator-sponsored programs, but

do not control their design or timing. Our collaboration programs include gene therapy candidates for Parkinson's disease, Sanfilippo B syndrome, Acute Intermittent Porphyria and amyotrophic lateral sclerosis.

#### *Bristol-Myers Squibb Collaboration*

On April 6, 2015, we entered into an agreement with Bristol-Myers Squibb, or BMS, that provides BMS exclusive access to uniQure's gene therapy technology platform for multiple targets in cardiovascular and other target-specific disease areas. The collaboration includes our proprietary congestive heart failure gene therapy candidate, which has demonstrated in advanced preclinical models that it can restore the ability to synthesize S100A1, a calcium sensor and master regulator of heart function, and increase survival rates after myocardial infarction. In addition, we will collaborate with BMS on up to nine additional gene therapy targets addressing a broad range of heart conditions and other target-specific disease areas. We will be responsible for discovery, preclinical development, and CMC, and will provide BMS our vector technologies and access to our industrial, proprietary insect-cell based manufacturing platform. uniQure will be responsible for CMC portions of regulatory filings and will co-operate with BMS in the preparation of all regulatory materials and interactions with regulatory authorities. BMS will be responsible for clinical development and all commercial activities across all programs.

The financial terms include guaranteed, near-term payments to us of at least \$97 million, including an upfront payment of \$50 million to be made at the closing of the transaction. The closing of the transaction is expected to occur in the second quarter of 2015 subject to Hart-Scott-Rodino clearance and customary closing conditions. An additional \$15 million payment is to be received following the selection of three additional collaboration targets, in addition to the S100A1 program, within three months of the closing. An initial equity investment in uniQure will be made for a number of shares that will equal 4.9% of the total number of shares outstanding following such issuance, at a purchase price of \$33.84 per share, or at least \$32 million in total. This investment is expected to be completed in the second quarter of 2015. BMS is also obligated to make an additional equity investment in uniQure for a number of shares that will equal 5.0% of the total number of shares outstanding following such issuance by December 31, 2015 and will be granted two warrants to acquire at its option up to an additional 10% equity interest, at a premium to market, based on additional targets being introduced into the collaboration. The parties have also agreed to enter into a supply contract, under which uniQure will undertake the manufacturing of all gene therapy products under the collaboration.

uniQure will be eligible to receive research, development and regulatory milestone payments, including up to \$254 million for the lead S100A1 therapeutic and up to \$217 million for each other gene therapy product potentially developed under the collaboration, assuming designation of all targets by BMS and achievement of all milestones. uniQure is also eligible to receive target designation fees, net sales based milestone payments and compensation on net product sales based on single- to double-digit percentages of net sales. See also "—Collaborations—Bristol-Myers Squibb Collaboration."

#### **Our Gene Therapy Development 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 and manufacturing platform. In some cases, we believe that the disease-specific gene and potentially the tissue-specific promoters will be the only components we need to change to target a new disease in a particular tissue. Combining this with the validated quality and safety of our manufacturing platform across our products, we believe that we can cross-reference data between

products, and thereby—on a case by case basis—we may be able to reduce the overall preclinical and clinical development activities required to obtain regulatory approval, and reduce significantly the overall development risk, time and cost.

The key components of our gene therapy approach are:

- **Therapeutic genes.** We design our gene therapies to deliver into the body's cells a transgene that encodes, or provides the blueprint for the expression of, a therapeutic protein. The transgene is carried in a gene cassette, or DNA sequence that encodes the specific genes and that includes DNA promoters that direct expression in specific tissues. We either develop our own gene cassettes or in-license them, often as part of our collaborations with academic research institutions and biotechnology and pharmaceutical companies. In-licensing gene cassettes provides us access to key intellectual property and allows us to build upon our collaborators' scientific expertise and financial investment, as well as their preclinical and, in some cases, clinical development efforts.
- **AAV-based vector delivery system.** We deliver the gene cassette to the target tissue using an engineered, non-replicating viral vector delivery system based on AAV, a common virus that affects humans but does not cause disease. We believe that AAV is the vector of choice for most *in vivo* gene therapy applications, such as ours, in which the functional gene is introduced directly into the patient's body. We use different variants, or serotypes, of AAV, 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 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 Molecular Therapeutics, or 4D, a 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 January 2015, we entered into a collaborative license agreement with Synpromics Limited to strengthen our technology platform with respect to therapeutic indications that require high-level therapeutic gene expression or comprise large therapeutic genes. We will exclusively own the results of this collaborative effort.

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 four 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. 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 believe that our manufacturing process, developed over ten years, demonstrates a high standard of safety and predictability. We have a manufacturing facility in Amsterdam, which has obtained EU regulatory approval for clinical and commercial grade production, and a facility in Lexington, Massachusetts, which we have recently equipped and which offers a 500-liter capacity that can be further expanded to 2,000L capacity when needed. We expect to commence internal GMP validation of this facility in the first half of 2015 and anticipate GMP production in the second half of 2015. We believe these two facilities will enable us to produce gene therapies cost effectively at commercial scale.

## 2014 Therapeutic Development Highlights

- In our hemophilia B program, we have received acceptance by the FDA of our investigational new drug application, or IND, and approval by the German Paul-Ehrlich-Institut, or PEI, of our Clinical Trial Application, or CTA, both in December 2014.
- Recently published follow-up data from a Phase I/II clinical trial conducted by St. Jude in the treatment of hemophilia B patients with functional human Factor IX gene, or hFIX, a blood clotting factor, have indicated that following a single intravenous infusion of vector in ten patients with severe hemophilia B (median duration post-treatment of 3.2 years):
  - patients receiving a high dose achieve larger than 5% sustained expression levels of the gene over many years after a single intervention;
  - this in turn reduces patients' need for prophylactic treatment, translating into a reduction by 96% of factor concentrate used; and
  - spontaneous bleeds were reduced in these high-dose patients by over 90%.

Our hemophilia B therapy uses the same functional gene as the St. Jude study.

- We announced our six-year follow-up data for patients treated once with Glybera. The data indicate that after a single administration, patients experienced reduction in both the frequency and severity of pancreatitis. We anticipate that the first patient will be treated with Glybera in the European Union in mid-2015.
- Our collaborator Digna Biotech completed a successful Phase I dose-escalation clinical trial testing an AAV5-PBGD gene therapy candidate, using our proprietary AAV5 viral vector, in patients with Acute Intermittent Porphyria, or AIP. Preliminary analysis of the one-year follow-up data indicates the safety of the therapy and successful transduction of liver cells with the PBGD gene.

## 2014 Business Highlights

- We acquired InoCard GmbH, an early-stage biotechnology company focused on the development of gene therapy approaches for cardiac diseases.
- We achieved our first published price for Glybera in Germany.
- We secured an additional \$10 million venture loan from Hercules Technology Growth Capital, Inc.
- We and Medison Pharma Ltd, Israel's leading international healthcare marketing group, entered into an exclusive distribution agreement under which Medison will market Glybera in Israel and the Palestinian Authority.
- We earned a total of €4.7 million in collaboration and licensing revenues.

## Product and Development Pipeline

The following table sets out the status of our approved product and each of our and our collaborators' development projects:

Product/ Product Candidate	Vector	Gene	Indication	Collaborator	Preclinical	Development Stage		Approved	Comments
						Phase I/II	Phase II/III		
Core Programs									
AMT-060	AAV5	Human Factor IX (hFIX)	Hemophilia B	Chiesi (in EU and other select countries)					<ul style="list-style-type: none"><li>• uniQure Phase I/II with AAV5 initiated first quarter of 2015</li><li>• Phase I/II trial by St. Jude using AAV8 &amp; uniQure's hFIX transgene ongoing</li></ul>
S100A1	AAV9	S100A1	Congestive Heart Failure	-					<ul style="list-style-type: none"><li>• We are currently preparing an EMA/FDA compliant pharma- cology/toxi- cology test plan</li></ul>
AAV5 Delivering Human Factor XIII	AAV5	Human Factor XIII	Hemophilia A	-					<ul style="list-style-type: none"><li>• Established proof of concept in non-human subjects</li></ul>
Validation Program									
Glybera (EU)	AAV1	Lipoprotein Lipase (LPL)	LPLD	Chiesi (in EU and other select countries)	We anticipate that the first patient will be treated in mid-2015				<ul style="list-style-type: none"><li>• We intend to launch a study in the first half of 2016 that will serve both as the post-approval study for the EMA and as the pivotal study for the FDA</li></ul>
Glybera (U.S.)	AAV1	LPL	LPLD	-					<ul style="list-style-type: none"><li>• Type C meetings with FDA in August and December 2013 and end-of-phase II-meeting in December 2014</li></ul>
Glybera (Rest of World)	AAV1	LPL	LPLD	-	Targeting markets that recognize EU marketing authorization				<ul style="list-style-type: none"><li>• Discussions with potential marketing collaborators ongoing</li></ul>
Collaborator Sponsored Programs									
AMT-021	AAV5	Porphobilin ogen Deaminase	Acute Intermittent Porphyria (AIP)	Digna Biotech (Licensor: CIMA)					<ul style="list-style-type: none"><li>• Phase I clinical trial by Digna Biotech completed</li></ul>
AMT-110	AAV5	NaGLU	Sanfilippo B Syndrome	Institut Pasteur (Sponsor: AFM)					<ul style="list-style-type: none"><li>• Phase I/II clinical trial with AAV5 conducted by Institut Pasteur. Data expected in the second half of 2015</li></ul>
AAV2 Delivering GDNF	AAV2	GDNF	Parkinson's Disease	UCSF (Funder & Sponsor: NIH)					<ul style="list-style-type: none"><li>• Phase I trial by UCSF/NIH using AAV2 &amp; GDNF transgene ongoing</li></ul>
	internal programs								
	collaborator sponsored programs								

## **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, either spontaneous or 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 hFIX as a result of mutations in the relevant gene. The presence of hFIX at greater than 1% of normal levels eliminates the risk of spontaneous bleeds. 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. Prophylactic protein replacement therapy is expensive, with an estimated annual cost ranging from \$300,000 to \$440,000 in the United States, but this can vary depending on disease severity and inhibitor status (this can be as high as \$1,126,000 for a patient with severe disease and inhibitors).

Hemophilia B affects approximately 1 in 20,000 live male births. A 2012 World Federation of Hemophilia, or WFH, survey identified 28,008 hemophilia B patients across 109 countries. An earlier WFH survey found that around 35% of identified hemophilia B patients were located in the European Union or the United States. 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.

### ***Our Development of AMT-060***

In collaboration with Chiesi, we are developing AMT-060, a gene therapy for the treatment of hemophilia B. The goal of our AMT-060 program is to restore blood clotting and to shift patients from the severe to the mild phenotype on a long-term and potentially curative basis through the delivery of the functional gene for hFIX into the patient's liver cells. 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.

AMT-060 consists of the 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 believe our AAV5 vector, exclusively licensed from NIH, carries a favorable safety and immunological profile compared with the AAV8 vector used by our competitors. We also believe that AMT-060 is currently the only gene therapy program using AAV5 vector for liver indications.

We initiated a Phase I/II clinical trial with this product candidate, described below, in the first quarter of 2015. Our collaborator St. Jude is currently conducting a Phase I/II clinical trial in this indication with an hFIX gene carried by an AAV8 vector. The vectors used by St. Jude are manufactured in a third party mammalian cell-based manufacturing process.

We filed an IND for AMT-060 with the FDA in December 2014 which has now been accepted. We also filed a CTA in Germany which was approved by the Paul-Ehrlich-Institute (PEI) in December 2014. We have pending approvals of CTAs and pending Environmental Safety approvals in Denmark, Sweden and the Netherlands.

### **Phase I/II Clinical Trial**

In the first quarter of 2015, we initiated our Phase I/II, open-label, uncontrolled, single-dose, dose-ascending multi-center clinical trial of AMT-060 in patients with severe or moderately severe hemophilia B. In this trial we are targeting sustained gene expression levels of over 5% with long term durability, a 90% reduction in both consumption of FIX replacement therapy and bleeding rates, as well as long-term safety. Our AMT-060 product candidate uses the same hFIX gene cassette being used in the St. Jude trial described below. One of our goals is to improve on the safety profile demonstrated by the St. Jude study through the use of our AAV5 vector, under exclusive license from NIH, manufactured using our validated baculovirus-based expression vector system. We also believe that AAV5 from the insect cell based manufacturing system may lead to a reduced incidence of organ toxicity compared with AAV8 from the mammalian based manufacturing system, potentially due to differences in the risk of induction of an immuneresponse. This outcome is supported by data from the ongoing clinical trial in AIP, described below, which uses the same dosage of the AAV5 vector as will be used in our hemophilia B trial, in which no immune response related liver toxicity occurred.

The key elements of our approved Phase I/II protocol are as follows:

- **Trial Population.** The trial will consist of two dosing cohorts, with five patients in each cohort. We will enroll male patients from multiple countries with either severe or moderately severe hemophilia B, but in either case with a severe bleeding phenotype. A maximum of two subjects with moderately severe hemophilia B will be enrolled per cohort.
- **Expedited Patient Enrollment.** Within each dosing cohort, we will allow a safety monitoring period of 24 hours between treating each patient. We will allow a period of 12 weeks for the first three patients between concluding treatment of the first cohort and commencing treatment of the second cohort.
- **Therapeutically Relevant Dosing Levels.** The lowest dosing cohort in our trial has been approved to receive a higher dose than the highest dosing cohort in the St. Jude trial.

### **St. Jude Clinical Trial**

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.

### **Preclinical Studies**

We have conducted 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.

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 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.

## **S100A1 for Cardiovascular Disease**

### ***Acquisition of InoCard***

On July 31, 2014 we acquired InoCard, an early-stage biotechnology company focused on gene therapy approaches for cardiac diseases. InoCard's integration into uniQure was completed in December 2014. InoCard's lead product against congestive heart failure (CHF) utilizes a cardiac-directed AAV-based gene therapy to reverse the expression deficit of the cardiomyocyte protein S100A1. InoCard has invested more than 15 years in understanding the S100A1 protein's unique role as a superordinate molecular regulator of the heart's calcium cycle that simultaneously controls contractility, energy metabolism, rhythm stability and growth of heart muscle. Lack of S100A1 protein expression is a hallmark of human CHF and drives disease progression and mortality. Pre-clinical studies by InoCard demonstrate that targeted restoration of diminished S100A1 protein levels reverse contractile dysfunction in human failing cardiomyocytes and translates into sustained improvement of cardiac performance and a notable effect on survival in a human-relevant pig chronic heart failure model.

Since our acquisition of InoCard, its two co-founders, Professors Katus and Most have joined us as Chairman of the Scientific Advisory Board, for Cardiovascular Diseases, and Managing Director of uniQure in Germany, respectively. In addition, it is agreed between Professor Katus and uniQure that he will be proposed for election to the board of directors of uniQure. Professor Katus is the Director of the Department of Internal Medicine III (Cardiology, Angiology and Pneumology) and Speaker of the Department of Internal Medicine at the Heidelberg University Hospital. He is an internationally renowned key opinion leader in clinical cardiology and heart failure research and has authored numerous scientific articles published in molecular and clinical journals. Professor Most is currently the General Manager of uniQure GmbH, Heidelberg, Germany, and head of the Molecular and Translational Cardiology Division at the Department of Cardiology at Heidelberg University Hospital. He holds an associate professorship of medicine at the Center for Translational Medicine at Thomas Jefferson University Medical College in Philadelphia and is an internationally renowned thought leader in cardiovascular molecular and translational research with extensive experience in the development and translation of molecular-targeted therapeutics for cardiac and vascular diseases. He serves as an

invited board member of the Cardiovascular Disease Scientific Committee of the European Society for Gene and Stem Cell Therapy and the Council on Basic Cardiovascular Sciences of the American Heart Association.

### ***CHF and Market Background***

InoCard's lead product, S100A1, is a cardiac-targeted gene therapy for advanced heart failure, designed to interrupt and reverse the natural downhill course of heart failure patients. We believe our S100A1 product candidate offers the prospect of a long-term, disease modifying benefit by improving survival and quality of life, which may prevent (re-) hospitalization due to acute cardiac decompensations, and reduce overall costs for clinical heart failure care.

It is estimated that there are approximately 20 million sufferers of congestive heart failure, or CHF, worldwide. The American Heart Association estimates that there are 5.8 million heart failure patients in the U.S. and projects that this number could be as high as 8.4 million by 2030.

S100A1 is targeted at advanced heart failure patients, and the treatment costs associated with advanced heart failure are high. It has been estimated that in Western, industrialized countries, 1% - 2% total annual healthcare expenditure is related to the care of patients with heart failure, with an almost exponential increase in cost of treatment associated with the severity of the patient's disease. In France, the Netherlands and Belgium, advanced heart failure patients represent 60% - 90% of the total heart failure spend.

Our S100A1 product candidate is designed to address this unmet medical need for a causal therapy that restores and stabilizes heart function. Researchers have observed diminished expression of the S100A1 protein in the failing heart. Our product's therapeutic effect restores the protein concentration and thereby allows the protein to re-establish its role as an integrated "master" regulator of the cardiac calcium-cycling pathway.

We expect that treatment would involve a one-time outpatient infusion in a cardiac catheterization laboratory, a standard procedure similar to undergoing a percutaneous coronary intervention or cardiac angiogram.

To further support our development of our S100A1 product candidate we have established a center of excellence for heart failure gene therapy research and development in the immediate proximity of the Heidelberg University Hospital's Heart Center.

### ***S100A1 Clinical Development to Date***

We have completed preclinical pharmacodynamic, pharmacokinetic and toxicological tests of our product candidate in a human-relevant experimental heart failure model under non-GMP/non-GxP conditions. The refined therapeutic formulation used in late preclinical development, and intended for human use, is scalable under GMP production. Our key preclinical data are as follows:

- S100A1 protein is downregulated in human CHF.
- Molecular analysis characterized the S100A1 protein as an upstream "master" regulator of the cardiomyocyte-calcium driven network by direct interaction and control of downstream molecular effectors of contractility, energy homeostasis, rhythm stability and growth regulation.
- S100A1 deficient hearts show accelerated progression to contractile failure, augmented cardiac remodelling, energetic breakdown and increase mortality after cardiac damage while elevated cardiomyocyte S100A1 protein levels are protective and prolong survival in mouse CHF models.
- Normalization of diminished S100A1 protein expression in human failing cardiomyocytes from explanted hearts by viral-based S100A1 gene therapy reversed contractile dysfunction, improved

energy production, protected against arrhythmias and reversed maladaptive growth. Low levels of the human S100A1 gene were sufficient to restore S100A1 protein expression and exert the profound therapeutic effect.

- Restoration of S100A1 protein expression deficit in a rat CHF by cardiac-targeted AAV-S100A1 gene therapy achieved long-term rescue of systolic and diastolic cardiac performance, reversed remodelling and was superior to the treatment with a clinically used CHF standard drug. Isolated cardiomyocytes from AAV-S100A1 treated rat heart showed superior systolic and diastolic performance.
- Cardiac-targeted delivery of AAV-S100A1 to failing hearts of domestic pigs by retrograde intravenous delivery resulted in widespread cardiac transduction and restoration of S100A1 protein expression that was contained to the heart. Long-term rescue of systolic and diastolic cardiac performance, improved energy metabolism, protection against maladaptive growth and tachyarrhythmias was achieved. Isolated cardiomyocytes from AAV-S100A1 treated pig heart showed superior systolic and diastolic performance.
- A 12 month follow up study unveiled a profound survival benefit in the pig CHF model by retrograde intravenous AAV-S100A1 delivery. We believe that outcome data obtained in this model are readily applicable to clinical trial design and endpoint selection. The following table indicates dramatically improved survival in the S100A1-treated pigs versus the placebo-treated animals (n=20 animals in each group).

We believe that our S100A1 product candidate in its current form generates the optimal S100A1 protein concentration for long-term therapeutic benefit. As such, we intend to use our product without further modification for use in clinical trials.

The long-term outcome of treatment with the S100A1 gene has been tested in large human-relevant animal models. In a population of domestic pigs with congestive heart failure, 12 months after treatment with the S100A1 gene using AAV9, 90% of the animals were still alive, while 90% of a control population of heart failure animals had died. We believe that outcome data obtained in human-relevant large animal therapy studies are readily applicable to clinical trial design and endpoint selection. The following table indicates dramatically improved survival in the S100A1-treated pigs versus the placebo-treated animals (n=20 animals in each group).

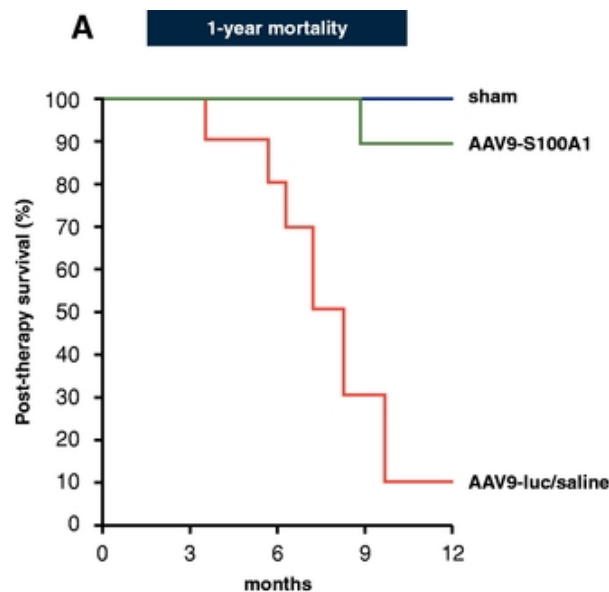


Image summarizing InoCard's data on improved survival of pigs with CHF and AAV-S100A1 treatment after one year follow-up (n=20 animals per group).

The remaining key milestones in our S100A1 product candidate's preclinical development include:

- Approval of an EMA/FDA-compliant pharmacology/toxicology test plan.
- Conclusion of GMP/GxP-compliant product testing and approval of the Phase 1b clinical trial protocol.

We are currently preparing the Phase 1b clinical trial protocol and expect to submit an IND application in the U.S. and a CTA in Germany by the end of 2016. The trial is planned to be held at 2-3 clinical centers of excellence in the U.S. and Germany seeking sequential FDA and EMA approval. We are transitioning S100A1 to our proprietary baculovirus-based manufacturing process and we intend that the product used in our Phase 1b trial will be manufactured using this process in our new Lexington facility. Through our relationship with the University of Heidelberg we are supported by the world leading experts and KOL's in the field of heart failure and these relationships will be important to securing strong leadership throughout the clinical trial program.

## **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.

## **Glybera for LPLD**

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.

LPLD is a serious, debilitating disease caused by mutations in the lipoprotein lipase, or LPL, gene, resulting in significantly 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. Life-long

adherence to this very restrictive diet is extremely difficult, with many individuals with LPLD remaining at increased risk for pancreatitis and other serious effects.

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.

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.

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

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 conduct post-approval clinical trials of Glybera.

The patient registry was put in place in May 2014, and we are currently in the process of designing the protocols for the post-approval clinical trials. We currently plan to enroll 12 patients with LPLD, along with a separate study in eight healthy volunteers. LPLD patients will receive a fixed dose of Glybera in a single series of intramuscular injections. 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 12 months and to complete enrollment into the study towards the end of 2016. The study has a two-year follow-up period. We will collect data on a broad range of biomarkers and clinically meaningful endpoints. The EMA has approved an initial protocol for this clinical trial in 12 patients. We are currently in the process of aligning protocols that will also address the requirements of the FDA, thereby bringing the required number of patients potentially up to 18, as described below.

We have developed an improved manufacturing process for Glybera, which addresses also our post-approval commitments, and will conduct consistency and comparability studies in respect of this process, which we expect to submit to the EMA in mid-2015.

#### ***Planned U.S. Program for Glybera***

We met with the FDA in type C meetings in August and December 2013 and in the form of an end-of-phase II meeting in December 2014 to establish 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 may provide data to support the bioactivity of Glybera. However, the FDA also advised that changes in chylomicron metabolism following a meal alone would not be recognized as an adequate biomarker for obtaining marketing approval in the United States at this stage, since it is not yet sufficiently established how restoration of post-prandial chylomicron metabolism translates into clinical meaningfulness. The FDA recommended that we identify and use additional disease manifestations of LPLD for which Glybera might have the best prospects for demonstrating a meaningful impact in the design of an adequate and appropriately controlled trial.

The aim of the discussion with the FDA is to adapt, but maintain, the details of the proposed EU post-approval trial and patient registry, and to identify how we might amend the protocol for the post-approval trial and patient registry so that they could also serve as a pivotal trial with a design that

addresses the FDA's requirements. Following these discussions, we are currently drafting a special protocol assessment, or SPA, for the FDA's review, which we expect to file during the first half of 2015. We hope that the SPA will be agreed by the FDA during the second half of 2015, and that we will be in a position to initiate a pivotal study of Glybera in the United States in the first half of 2016.

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

We expect to treat the first patient with Glybera, in Germany in mid-2015, through our collaboration with Chiesi, with treatments in other European markets to follow. We and Chiesi are working together through a joint commercialization committee to, among other things, plan and execute the market roll-out strategy and pre-launch preparations in other selected countries in the European Union covered by our agreement with Chiesi, incorporating our business model for the commercialization of a therapy administered in a one-time intervention. We and Chiesi have also built new models for product pricing and reimbursement, have expanded key opinion leader relationships, continue to identify centers of excellence in European countries, and have developed physician and patient education and patient access programs. The Glybera pricing and reimbursement, or P&R, dossier has been submitted in several European countries. Chiesi is currently in discussion with the appropriate authorities at the country level, focusing on several EU countries in parallel.

#### ***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. In Germany, Glybera has received its first published price. The pricing model chosen for Glybera in Germany is a one-time payment of EUR 41,000 per vial. A single treatment for patients weighing 60-70 kilograms requires 20-24 vials.

This price is still subject to change following an assessment by the German Federal Joint Committee (Gemeinsamer Bundesausschuss, or G-BA). The G-BA is expected to publish the results of the assessment by the end of April 2015. The G-BA assessment will be followed by negotiations with the Statutory Health Insurance (SHI) and only at the end of this process will a final price be set.

On April 1, 2015, a list price was established in the United Kingdom at a similar level to the list price in Germany. 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. 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.

#### ***Commercial Preparation and Roll-Out***

Chiesi has identified, and is continuing the process of identifying, centers of excellence in each of the five largest EU markets (France, Germany, Italy, Spain and the United Kingdom) where Glybera will be administered, as part of the pre-launch roll-out. Chiesi is working closely with these centers to, among other things, establish patient registries and prepare treatment procedures for LPLD patients. Chiesi is developing a strategy to facilitate patient referrals to these centers, in part through broader educational efforts and outreach to relevant medical practitioners and other key stakeholders 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 educational materials regarding LPLD, Glybera 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 the commission of a now completed 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.

### ***Summary of Glybera Clinical Development Program***

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 two retrospective case note reviews of 19 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. In the three clinical studies, we did not observe a statistically significant reduction in fasting triglyceride levels beyond 12 weeks, which was the primary efficacy 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. Patients were observed prior to treatment with Glybera, and at 14 weeks and 52 weeks following treatment. We observed a consistent and significant improvement in the appearance and removal of newly formed chylomicrons in the blood in all five patients measured at week 14 after treatment and three out of five patients measured at week 52 after treatment.

The case note reviews 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.

### **Collaborator sponsored programs**

As part of our strategy we are collaborating with third parties and are sponsoring early state 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 designs 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 summarized below:

#### ***AMT-021 for Acute Intermittent Porphyria***

We 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 former collaborator Digna Biotech has completed a Phase I clinical trial of AMT-021 in eight patients in Spain. The collaboration achieved its primary goal in completing a successful Phase I study, thereby establishing the preliminary safety profile of liver-directed gene therapy with AAV5 that we expect will support future clinical studies.

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

Digna Biotech conducted a multicenter, open label, prospective, interventional, single dose, dose-escalation Phase I clinical trial to investigate the safety and tolerability of AMT-021 in eight patients with severe AIP. Digna Biotech conducted this clinical trial at two sites in Spain. There were four dosing cohorts in the trial, with two patients per cohort. Digna Biotech monitored all patients for one year following treatment. The primary objective of this Phase I clinical trial was to assess the safety of systemic administration and to determine the maximum tolerated dose of AMT-021. Secondary objectives included measuring urinary levels of toxic metabolites to determine whether these metabolites can be used as a biomarker of potential treatment effect. Preliminary analysis of the one-year follow-up data indicates the safety and successful transduction of liver cells with the PBGD gene. Key findings were as follows:

- There were no liver enzyme perturbations.
- There was no evidence of cellular immune response against the AAV vector or the PBGD gene.
- Vector genomes were detected in patients' liver biopsies obtained under one year after vector injection, indicating that AMT-021 sustainably transduced human liver cells.

Digna has advised us that there were four serious adverse events in this trial; however the events were 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 did not observe 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 Phase I clinical trial, we are currently assessing whether higher dose levels or a more potent vector may be the relevant next step in the project. Under our consortium agreement with Digna Biotech and the other consortium members, we have an exclusive right to use all data related to the 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 N acetylglucosaminidase, or NaGLU, gene. Institut Pasteur is currently conducting a Phase I/II clinical trial of AMT 110 in four patients in France, where recruitment has commenced in October 2013. 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 one-year follow-up data are expected in the second 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.

### *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 one-year follow-up data are expected in the second half of 2015. This Phase I/II clinical trial is being conducted in Paris, France, fully recruited and with a



follow-up period of one year for each patient. The follow-up duration is currently being extended as an amendment to the protocol. 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 receive immunosuppressive therapy on an ongoing basis, to prevent an immune response to either the AAV vector capsid or the expressed protein. The primary objective is to evaluate biomarkers of efficacy, clinical and radiological markers of benefit as well as the biological safety of the proposed treatment. The secondary objective is to collect data that could inform further clinical studies.

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

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

- Rodents displayed no signs of toxicity at seven days, three months or six months after treatment despite administration of up to 37 times the level of dosage required for human patients.
- Biodistribution studies in rodents indicated no differences between those following an immunosuppressant treatment course and those that were not, and shedding from major organs over time.
- 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*

We and our collaborator the University of California at San Francisco, or UCSF, are developing a gene therapy for Parkinson's disease, a progressive neurodegenerative disorder. UCSF is collaborating with the NIH to conduct a Phase I 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. The trial will involve 24 patients across four dosing cohorts (six patients per cohort), and treatment of the first of these cohorts is now complete. 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 development program. Based on the results of the UCSF program, we may decide to develop an AAV2-based gene therapy containing the GDNF gene manufactured with our insect cell-based manufacturing process.

#### *Potential Additional Pipeline Programs*

We are also conducting early-stage preclinical research into a number of other potential applications of our technologies. Currently these programs focus on utilizing AAV5 in liver and CNS indications, including Huntington's disease. Based on defined criteria for indications that we believe most likely to be well suited to our gene therapy approach, we have prioritized approximately six additional target diseases. In addition, we have an ongoing cardiovascular-specific evaluation through which we have identified three targets beyond S100A1. 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 preclinical or clinical studies. We are also conducting preclinical research into potential applications of our technology in transcription silencing, also called post transcriptional gene silencing. This is a biological process in which RNA molecules inhibit gene expression, typically by causing the destruction of specific miRNA molecules.

## **Collaborations**

### ***Overview of our Collaboration Strategy***

Our collaboration strategy is based on establishing efficiencies by collaborating at three key stages in the development of our therapies: first, with leading early-stage companies and academics conducting ground-breaking research into new technologies and therapies relevant to our core business and therapeutic areas of interest; second with pharmaceutical companies that are able to contribute late-development stage expertise and further investment; and third with pharmaceutical companies with a view to profitably commercializing our approved products. A summary of our key current collaborations is set out below.

### ***Bristol-Myers Squibb Collaboration***

On April 6, 2015, we entered into a series of agreements with BMS, a publicly traded pharmaceutical company, regarding a collaboration that provides BMS with exclusive access to our gene therapy technology platform for multiple targets in cardiovascular and potentially other diseases. As part of this collaboration, BMS has also agreed to make an equity investment in our company.

### ***Collaboration and License Agreement***

Under our Collaboration and License Agreement with BMS, we will provide BMS with exclusive access to our proprietary gene therapy program for multiple targets in cardiovascular diseases. The collaboration includes our proprietary congestive heart failure gene therapy program, which has demonstrated in advanced preclinical models that it can restore the heart's ability to synthesize the protein S100A1, a calcium sensor and master regulator of heart function. In addition, we will collaborate with BMS on up to nine additional gene therapy targets addressing a broad range of cardiovascular and other target-specific areas.

Pursuant to the agreement, we are responsible for leading discovery efforts and for manufacturing clinical and commercial supplies using our vector technologies and our industrial, proprietary insect-cell-based manufacturing platform. BMS is responsible for leading development and regulatory activities across all programs and is responsible for all research and development costs. BMS is also solely responsible for the commercialization of all products from the collaboration. We have also agreed to enter into a supply contract, under which we will undertake manufacturing of all gene therapy products under the collaboration.

We will receive an upfront payment of \$50 million at the closing of the collaboration, which is expected to occur in the second quarter of 2015, and will receive an additional \$15 million payment for the selection of three collaboration targets, in addition to S100A1, to be made within three months of the closing of the collaboration. We will be eligible to receive additional payments for further designation of new collaboration targets and upon the achievement of research, development and regulatory milestones, including up to \$254 million for the lead S100A1 therapeutic and up to \$217 million for each other gene therapy product potentially developed under the collaboration. We will also be eligible to receive net-sales-based milestone payments and tiered single to double-digit royalty payments on product sales.

## *Equity Agreements*

As part of the collaboration, pursuant to a Share Subscription Agreement, BMS will acquire a number of our ordinary shares equal to 4.9% of our outstanding ordinary shares following such issuance, at a purchase price of \$33.84 per share, and will acquire a number of additional ordinary shares equal to 5.0% of our outstanding ordinary shares following such issuance before December 31, 2015, at a 10% premium to the volume-weighted average price for the twenty (20) trading days ending on the date that is five (5) trading days prior to such acquisition.

We have also granted BMS two warrants, pursuant to each of which BMS may at its option acquire an additional number of shares equal to up to 5.0% of our outstanding ordinary shares (10.0% in the aggregate) immediately after each such issuance, at a premium to market. The exercise of each warrant is conditioned upon the designation of a specified number of additional collaboration targets and payment of related fees by BMS, as well as a minimum number of collaboration programs under development.

The total number of ordinary shares that may be acquired by BMS pursuant to the Share Subscription Agreement and exercise of both warrants is equal to 19.9% of the total number of ordinary shares outstanding following such issuances. In the event that our shareholders do not specifically approve these issuances at our 2015 annual general meeting, however, the number of shares issuable to BMS pursuant to exercise of the warrants would be limited such that the aggregate number of shares issuable under these arrangements does not exceed 19.9% of the number of ordinary shares outstanding immediate prior to the signing of these agreements.

We also entered into an Investor Agreement with BMS regarding the rights and restrictions relating to the ordinary shares to be acquired by BMS. We have granted BMS certain registration rights that allow BMS to require us to register our securities beneficially held by BMS under the Exchange Act. BMS may make up to two such "demands" (or three, in the event that either warrant is exercised) for us to register the shares, provided that we may deny such demand if (i) the market value of the shares to be registered is less than \$10 million (provided however, if BMS holds less than \$10 million worth of our shares, we must comply with their demand for registration), (ii) we certify to BMS that we plan to effect a registration within 120 days of their demand or we are engaged in a transaction that would be required to be disclosed in a registration statement and that is not reasonably practicable to be disclosed at that time, or (iii) we have already effected one registration statement within the twelve months preceding BMS's demand for registration. In addition, upon the occurrence of certain events, we must also provide BMS the opportunity to include the shares they hold in any registration statement that we effect independent of any demand registration.

We have also granted BMS certain information rights under the Investor Agreement, although these requirements may be satisfied by our public filings required by U.S. securities laws.

Pursuant to the Investor Agreement, without our consent, BMS may not (i) acquire a number of shares such that the number of shares that BMS beneficially holds is greater than the percentage acquired, or which may be acquired, after giving effect to each of the tranches under the Share Subscription Agreement and the two warrants; (ii) propose, offer or participate in any effort to acquire us or one of our subsidiaries; (iii) propose, offer or participate in a tender offer for our shares or any exchange of shares that would effect a change of control of our company; (iii) seek to control or influence our governance or policies; (iv) join or participate in any group regarding the voting of our ordinary shares; or (v) take certain other similar actions. BMS may still, among other things, make a non-public, confidential proposal to enter into a business combination or similar transaction with our company. These "stand still" restrictions will terminate upon the occurrence of certain events including, but not limited to, the acquisition of a certain material number of shares by a third party, if we enter into a merger agreement or similar transaction with a third party, or upon the passage of a defined

period of time subsequent to the acquisition of shares pursuant to the Share Subscription Agreement or the warrants.

BMS is also subject to a "lock-up" pursuant to the Investor Agreement. Without our prior consent, BMS may not sell or dispose of its shares until the later of (i) the fourth anniversary of the purchase of the first tranche of shares pursuant to the Share Subscription Agreement (or fifth anniversary if the Collaboration Agreement is extended), or (ii), in respect of each ordinary share acquired pursuant to the Share Subscription Agreement and the warrants, the first anniversary of issuance of each such ordinary shares. However, this "lock-up" may terminate sooner in the event the Collaboration Agreement is terminated.

The Investor Agreement also requires BMS to vote all of our ordinary shares it beneficially holds in favor of all items on the agenda for the relevant general meeting of shareholders of our company as proposed on behalf of our company, unless, in the context of a change of control or similar transaction, BMS has itself made an offer to our company or our supervisory or management boards in connection with the transaction that is the subject of the vote, in which case it is free to vote its shares at its discretion. This voting provision will terminate upon the later of the date on which BMS no longer beneficially owns at least 4.9% of our outstanding ordinary shares, the closing of a transaction that provides BMS exclusive and absolute discretion to vote our shares it beneficially holds, or the termination of the Collaboration Agreement for breach by us.

### ***Early-Stage Collaborations***

#### ***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, to develop, make, use and sell certain AAV vectors and products containing such AAV vectors and gene constructs, for delivery of such gene constructs to CNS or liver cells for the diagnosis, treatment, palliation or prevention of any disease or medical condition. 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 established a laboratory to identify next generation AAV vectors. In addition, in connection with our entry into this collaboration, Dr. Schaffer became a member of our Supervisory Board.

We are currently funding a three-year research collaboration, which can be extended at our option for an additional year, being conducted under a mutually agreed research plan. We are entitled to select a specified number of AAV variants from the research collaboration. We have exclusive rights to further research, develop, manufacture and commercialize the selected AAV variants, as well as AAV vectors and products containing such AAV variant and gene constructs, or licensed products, and, during the research collaboration and for the term of the agreement, 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 and gene constructs, for delivery of gene constructs to CNS or liver cells for the diagnosis, treatment, palliation or prevention of any disease or medical condition.

Our research collaboration with 4D is guided by a joint research steering committee. Under the agreement, we have made 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. Both of these payments were made in the first quarter of 2014. Our payment obligations under the agreement include the research collaboration funding described above as well as

payments for the achievement of specified preclinical, clinical and regulatory milestones of up to \$5,000,000 for each licensed product that we develop under the collaboration, and, for each licensed product, each indication. 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 also pay 4D a low to upper-low 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.

#### *Treeway*

In January 2015, we entered into a license and collaboration agreement with Treeway B.V., a private company founded by entrepreneurs Bernard Muller and Robbert Jan Stuit, both diagnosed with amyotrophic lateral sclerosis, or ALS, to develop a gene therapy treatment for ALS. Under the terms of the agreement, we have granted Treeway an exclusive license in this field to uniQure's relevant AAV5 viral vector and GDNF (Glial cell-derived neurotrophic factor) intellectual property. Treeway is responsible for the preclinical and clinical development of the ALS gene therapy treatment. We will provide Treeway with our development and manufacturing capabilities and will further collaborate with Treeway on ALS gene therapy development. We and Treeway expect to jointly commercialize any resulting ALS gene therapy with defined geographical rights for commercialization assigned to each company.

#### *Synpromics*

In January 2015, we entered into an agreement with Synpromics, a United Kingdom-based biotechnology company, pursuant to which we intend to jointly fund research relating to the development of optimized viral promoters. Under the agreement, we have agreed to fund a specific testing program on liver promoters, with payments based on the achievement of specified milestones. Following the conclusion of the non-clinical testing phase, further milestones and payments have been agreed through the clinical phase of development and commercialization of products consisting of promoters developed under this agreement.

#### ***Chiesi Commercialization and Development Agreement***

We have entered into an agreement with Chiesi Farmaceutici S.p.A., a family-owned Italian pharmaceutical company with 2013 worldwide revenues of approximately € 1.2 billion, for the co-development and commercialization of our hemophilia B program. We have retained full rights in the United States, Canada and Japan under this agreement. We received a €15.0 million upfront payment under this agreement, as well as a €14.0 million investment in our ordinary shares, both in July 2013. This agreement provides us with research funding for further development of our hemophilia B product candidate, and further provides that 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 collaboration with Chiesi is guided by a joint steering committee, a joint development committee and a joint commercialization committee.

#### *Therapy Commercialization*

We have entered into an agreement with Chiesi for the commercialization of Glybera for LPLD. We have retained full rights in the United States, Canada and Japan under this agreement. 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 from Chiesi for the quantities of Glybera we manufacture and supply to them. 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 €5.8 million as of December 31, 2014.

## **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.

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.

Our patent portfolio includes the following patent families:

- 16 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:

- 8 issued U.S. patents;
- 6 granted European Patent Office patents;
- 3 pending PCT patent application;
- 6 pending U.S. patent applications;
- 8 pending European Patent Office patent applications; and
- 19 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 two issued patents in the United States, an issued patent in Japan and pending applications in the United States 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 own a method of a manufacturing patent family relating to our 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 including the United States and Japan. The standard 20-year term for patents in this family will expire in 2028.

### *S100A1*

InoCard holds patents regarding the S100A1 lead compound in our S100A1 product candidate in heart and skeletal muscle diseases. The patents have been granted in Europe, Canada, Japan and the US, the term of which will expire in 2020.

Since our acquisition of InoCard, Professors Katus and Most have joined us as Managing Director of uniQure in Germany and Chairman of the Scientific Advisory Board, for Cardiovascular Diseases, respectively.

InoCard has developed and the Technology Transfer, Heidelberg University has filed a "second medical use" patent application relating to the therapeutic window and effective dosages of S100A1 in heart disease. An exclusive option agreement was signed, and was transferred to us through our acquisition of InoCard. We expect that this process could extend the exclusivity period until 2034.

Professors Katus and Most have developed and the Technology Transfer, Heidelberg has filed, patents applications relating to InoCard's early-stage development of systemic S100A1 peptide therapy for mild heart failure and skeletal muscle diseases. An option agreement was finally negotiated between InoCard and Technology Transfer, and was transferred to us through the acquisition of InoCard.

### *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., subsequently acquired by Sanofi, a patent family co-owned by UBC and Sanofi that relates to the use of AAV-LPL vectors for LPL-deficiency, including issued patents in Europe and other jurisdictions and two pending U.S. patent applications. The standard 20-year term for patents in this family will expire in 2015. Product protection will be extended by this license until 2020 in those European countries where a supplementary protection certificate, or SPC, will be granted. In some European countries, Sanofi has applied for SPCs on the



basis of their patent EP0946741, and our market authorization for Glybera. In Italy, an SPC has been granted to Sanofi by the Italian Patent and Trademark Office, or PTO, but we believe that not all of the relevant information was made known to the PTO at that time. Accordingly we believe that the Glybera product produced by our proprietary manufacturing methods does not infringe the claims presented in EP0946741.

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, Japan and elsewhere, as well as a pending application in Europe. 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.

We also own a family of patent applications relating to a proprietary baculovirus filtration process. This family includes pending applications in the United States, Europe, Japan and other jurisdictions. 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.

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.

#### *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 two in Europe. The U.S. 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 granted patents in the United States, Europe and elsewhere. The family also includes pending applications in other jurisdictions. 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.

#### ***4D Molecular Therapeutics***

In January 2014, we entered into a collaboration and license agreement with 4D Molecular Therapeutics 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. Our payment obligations under this license agreement consisted of a one-time up-front payment of \$200,000 which we have paid.

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 three years, to identify next generation AAV vectors. We will also be 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 609,744 ordinary shares in connection with this collaboration, and will recognize resulting share-based payment expense over 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.

#### ***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 \$328,684 in amendment and sublicense payments, together with a follow-on amendment payment of \$100,000 in October 2014. 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 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 \$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 low-twenties 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 low-twenties 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 collaborator, 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 collaborator plus an equivalent percentage of the price we invoice the commercialization collaborator 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 collaborator. 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 collaboration 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.

*Asklēpios 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 and 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, 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 Aplicada, or FIMA, the members of a collaborative research consortium known as UTE CIMA, and Proyecto de Biomedicina CIMA S.L., or Proyecto, which superseded several prior agreements amongst such parties. We refer to Digna Biotech, FIMA, UTE CIMA and Proyecto collectively as the CIMA Parties. Under the license agreement, Proyecto granted us an exclusive, worldwide license, with a right to sublicense, under its interest in patent rights we jointly own with Proyecto relating to PBGD gene therapy to use, develop, make, have made and commercialize products using the licensed patent rights. In addition, UTE CIMA granted us a non-exclusive, worldwide license, with the right to grant sublicenses, under certain patent rights, know-how and materials required for the use, development, manufacture or commercialization of products covered by our exclusive license from Proyecto in the gene therapy field.

We have agreed to pay Digna Biotech royalties equal to a mid-single digit percentage of net sales, if any, by us or our affiliates of licensed products covered by our exclusive license and a sub-teen

double-digit percentage share of net revenues we receive from our sublicensees. Digna Biotech is responsible for apportioning the amounts we pay Digna Biotech amongst the CIMA Parties.

Under the agreement we have to use commercially reasonable efforts to further develop, manufacture and commercialize licensed products as soon as reasonably practicable.

The agreement will remain in effect until our payment obligations expire or we or another party exercise our rights to terminate it. A party may terminate the agreement in circumstances relating to another party's insolvency or bankruptcy or if our agreement under which Digna Biotech is conducting a Phase I clinical trial of AMT-021 terminates. We may terminate this agreement for convenience, subject to a specified notice period. If Digna Biotech terminates the license agreement for breach or insolvency, we or Digna Biotech terminate the license agreement because our other agreement with Digna Biotech terminates other than for breach or insolvency of Digna Biotech or if we terminate the license agreement for convenience, the CIMA Parties will have the exclusive right to use the patent rights we jointly own with Proyecto that were exclusively licensed to us to further develop and commercialize licensed products for the treatment or prevention of AIP without financial obligations to us.

### ***Parkinson's disease***

#### *Amgen*

In 2010, we entered into a license agreement with Amgen, Inc. which superseded a prior 2008 agreement. Under the license agreement, Amgen granted us an exclusive, worldwide license, with a right to sublicense, to patents and know-how relating to GDNF to research, develop, make, use, offer for sale, sell, import, export and otherwise exploit gene therapies capable of delivering GDNF, the gene encoding GDNF, or any fragment of GDNF that has specified functional activity, which we refer to as GDNF products. The license exclusivity, and our obligation to make the revenue sharing payments described below, with respect to a given GDNF product in a given country expires on the later of expiration of the last-to-expire licensed patent in such country that covers such GDNF product and the tenth anniversary of the first commercial sale of such GDNF product in such country. Thereafter the license would become non-exclusive with respect to that GDNF product in that country.

We have agreed to pay Amgen revenue sharing payments equal to a sub-teen double-digit percentage of net revenues, if any, that we receive from our sales of GDNF products, from granting sublicenses under the intellectual property licensed from Amgen or from granting licenses under certain of our intellectual property rights. Upon receipt of the first marketing approval anywhere in the world for the first GDNF product we have also agreed to pay Amgen a one-time milestone fee of the greater of \$10 million or a sub-teen double digit percentage of any milestone payments we receive from third parties with respect to receiving such approval.

We agreed to use reasonably diligent efforts to develop at least one GDNF product and seek to obtain regulatory approvals for this GDNF product in the United States and the European Union, and to commercialize it.

We granted Amgen an option to negotiate an exclusive license from us to research, develop, make, use, offer for sale, sell and otherwise exploit GDNF products in the United States, Mexico and Canada. Amgen may exercise the option within a specified period following completion of the first Phase II clinical trial of the first GDNF product we develop. If Amgen exercises the option but we and Amgen do not execute a definitive agreement to grant these rights to Amgen within a specified period of time, we retain these rights but may not grant development or commercialization rights to a third party in these North American countries on financial terms less favorable to us than those last offered by Amgen.



The agreement will remain in effect until either we or Amgen exercise our rights to terminate it. We may terminate the agreement for convenience at any time subject to a specified notice period. If we terminate the agreement for convenience, or if Amgen terminates the agreement due to our uncured material breach, rights to GDNF products will revert to Amgen. As part of such reversion, if Amgen requests, we have agreed to grant Amgen an exclusive, worldwide license under our relevant intellectual property rights so that Amgen can research, develop, make, use, offer for sale, sell and otherwise exploit GDNF products, subject to a specified revenue sharing and a one-time regulatory milestone payment from Amgen to us.

#### *UCSF*

In 2012, we entered into a data license agreement with the University of California in San Francisco, or UCSF, related to UCSF's rights to the clinical trial data from a Phase I/II clinical trial, sponsored by the NIH, and that UCSF is conducting, of a product candidate consisting of an AAV2 vector carrying the GDNF gene, and to certain related preclinical data and know-how. Under the data license agreement, UCSF granted us a non-exclusive license, with a right to sublicense, to research, develop, make, use, offer for sale, sell and otherwise exploit pharmaceutical products containing or consisting of an AAV2 genetic construct encoding GDNF, or any fragment of GDNF that has specified functional activity, for the therapeutic, palliative and prophylactic treatment of Parkinson's disease in humans. During the term of the data license agreement, UCSF has agreed not to grant to any other for-profit entity any of the rights granted to us thereunder, except under specified circumstances involving a breach of our diligence obligations described below.

Payment obligations under the agreement include a one-time, up-front payment of \$300,000, which we have paid, a royalty equal to a low single-digit percentage of our net sales, if any, of products that are identified or developed through material use of the data licensed from UCSF, or identified products, as well as third party license fees with the percentage due to UCSF ranging from a low double-digit percentage for earlier-granted sublicenses to a low single-digit percentage for later-granted sublicenses. Our obligation to pay UCSF earned royalties with respect to a given country begins on the first commercial sale of an identified product in such country, and our obligation to pay earned royalties and third-party license fees expires on the tenth anniversary of such first commercial sale, after which the data license will become perpetual, non-exclusive, fully paid-up, and royalty-free in such country.

The UCSF agreement has been amended such that other obligations we had agreed to complete by specified dates are now related to the receipt of an interim report of the ongoing clinical study at NIH, including obligations to deliver to UCSF 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 collaborator. 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.

## **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 AGTC, AGTC, Asklepios, Audentes Therapeutics, Avalanche, Baxter, bluebird bio, Celladon, Dimension/Regen X, Isis, Oxford Biosciences, Sangamo BioScience, Spark Therapeutics and Voyager, 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 Amgen, Baxter, Bayer, Biogen Idec, BioMarin, Genzyme, Novartis, Novo Nordisk, Pfizer, Shire, 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.

Many of our current or potential competitors, either alone or with their collaborators, 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.

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.

## **Government Regulation and Reimbursement**

Government authorities in the United States, European Union and other countries extensively regulate, among other things, the approval, research, development, preclinical and clinical testing, manufacture (including any manufacturing changes), packaging, storage, recordkeeping, labeling, advertising, promotion, distribution, marketing, post-approval monitoring and reporting, reimbursement, 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. For other countries outside of the United States and the European Union, marketing approval and pricing and reimbursement requirements vary from country to country. If we fail to comply with applicable regulatory requirements, we may be subject to, among other things, fines, refusal to approve pending applications, suspension or withdrawal of regulatory approvals, product recalls, seizure of products, operating restrictions and criminal prosecution.

### ***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 guidance implementing these laws. Obtaining regulatory approvals and ensuring compliance with applicable statutes and regulatory requirements entails the expenditure of substantial time and financial resources, including payment of user fees for applications to the FDA. All of our current product candidates are subject to regulation by the FDA as biologics. An applicant seeking approval to market and distribute a new biologic in the United States must typically undertake the following:

- completion of preclinical laboratory tests, animal studies and formulation studies in compliance with the FDA's current Good Laboratory Practice, or cGMP, 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, 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;
- approval of the BLA by the FDA, in consultation with an FDA advisory committee, if deemed appropriate by the FDA;
- 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; and
- compliance with any post-approval commitments, including Risk Evaluation and Mitigation Strategies, or REMS, and post-approval studies required by the FDA.

## ***Human Clinical Studies in the US 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 in the US 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. The protocol and informed consent documents must also be approved by an IRB. The FDA, an IRB, 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. Information about certain clinical trials, including results, must be submitted within specific timeframes for listing on the 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.

## ***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 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.

### ***FDA Programs to Expedite Product Development***

The FDA has several programs to expedite product development, including fast track designation and breakthrough therapy designation. 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. The benefits include greater interactions with the FDA, eligibility for accelerated approval based on a surrogate endpoint, eligibility for priority review of the BLA, and rolling review of sections of the BLA. The 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.

### ***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. The submission of a BLA is subject to an application user fee, currently exceeding \$2.3 million, and the sponsor of an approved BLA is also subject to annual product and establishment user fees, currently exceeding \$110,000 per product and \$569,000 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 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 that 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 designation receives the first FDA approval, it will be granted 7 years of marketing exclusivity, which means that the FDA may not approve any other applications for the same product for the same indication for seven years, unless clinical superiority is demonstrated in a head-to-head trial. 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. The FDA has 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 provides for the attachment of an additional six months of marketing protection to the term of any existing regulatory exclusivity in the US, 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.

## ***FDA Regulation of Companion Diagnostics***

We may seek to develop companion diagnostics for use in identifying patients that we believe will respond to our gene therapies. FDA officials have issued draft guidance to 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 companion diagnostic 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 device, then the FDA generally will require approval or clearance of the diagnostic device at the same time that the FDA approves the therapeutic product.

## ***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 EU member states. 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 an EU 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 CTA, 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—EU 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, and advanced therapy medicinal products as defined in Regulation (EC) No 1394/2007, as amended. 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, as amended, also fall within the mandatory scope of the centralized procedure. Because of 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 are expected to 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. Guidance on the factors that the EMA will consider in relation to the development and evaluation of ATMPs have been issued 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 and maintain approval for any of our product candidates. The maximum timeframe for the evaluation of an MAA under the centralized procedure is 210 days after receipt of a valid application.

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 authorized 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 reassessment 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, preclinical tests and clinical trials. The EMA has also issued guidelines for a comprehensive comparability exercise for biosimilars, and for specific classes of biological products.

Additional rules apply to medicinal products for pediatric use under Regulation (EC) No 1901/2006, as amended. 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 an orphan medicinal product 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, as amended, 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 is prohibited. 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 collaborators, provided that this delegation is appropriately documented and the MAH maintains legal responsibility and liability.

The obligations of an MAH include:

- *Manufacturing and Batch Release.* MAHs should guarantee that all manufacturing operations comply with relevant laws and regulations, applicable good manufacturing practices, with the product specifications and manufacturing conditions set out in the marketing authorization and that each batch of product is subject to appropriate release formalities.
- *Pharmacovigilance.* MAHs are obliged to establish and maintain a pharmacovigilance system, including a qualified person responsible for oversight, to 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 their 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 their medicinal products to healthcare professionals, regulators and patients.
- *Legal Representation and Distributor Issues.* MAHs are responsible for regulatory actions or inactions of their distributors and agents.
- *Preparation, Filing and Maintenance of the Application and Subsequent Marketing Authorization.* MAHs must maintain appropriate records, comply with the marketing authorization's terms and conditions, fulfill reporting obligations to regulators, submit renewal applications and pay all appropriate fees to the authorities.

We hold the marketing authorization under exceptional circumstances granted for Glybera in the European Union and we may hold any future marketing authorizations granted for our product candidates in our own name, or appoint an affiliate or a collaborator to hold marketing authorizations on our behalf. Any failure by an MAH to comply with these obligations may result in regulatory action against an MAH and ultimately threaten our ability to commercialize our products.

#### *Reimbursement*

In the European Union, the pricing and reimbursement mechanisms by private and public health insurers vary largely by country and even within countries. In respect of the public systems reimbursement for standard drugs is determined by guidelines established by the legislator or responsible national authority. The approach taken varies from member state to member state. Some jurisdictions operate positive and negative list systems under which products may only be marketed once a reimbursement price has been agreed. Other member states allow companies to fix their own prices for medicines, but monitor and control company profits and may limit or restrict reimbursement. The downward pressure on healthcare costs in general, particularly prescription drugs, has become very intense. As a result, increasingly high barriers are being erected to the entry of new products and some of EU countries require the completion of studies that compare the cost-effectiveness of a particular product candidate to currently available therapies in order to obtain reimbursement or pricing approval. Special pricing and reimbursement rules may apply to orphan drugs. Inclusion of orphan drugs in reimbursement systems tend to focus on the medical usefulness, need, quality and economic benefits to patients and the healthcare system as for any drug. Acceptance of any medicinal product for reimbursement may come with cost, use and often volume restrictions, which again can vary by country. In addition, results-based rules of reimbursement may apply.

#### *Orphan Drug Regulation*

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 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 an EU-wide community marketing authorization in respect of an orphan drug is granted pursuant to Regulation (EC) No 726/2004, as amended, 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.

### **C. Organizational Structure**

uniQure N.V. has eleven direct and indirect wholly owned subsidiaries, each listed in Note 1 to the financial statements which form part of this annual report and in Exhibit 8.1 to this annual report. Our principal operating companies are uniQure biopharma B.V., a Netherlands company, uniQure, Inc., a Delaware corporation, and uniQure GmbH, a German limited company.

### **D. Property, Plant and Equipment**

We lease a facility of approximately 26,000 square feet from the AMC, located at Meibergdreef in Amsterdam, the Netherlands, which forms our headquarters and principal laboratories, and also houses our manufacturing facility which the EMA has approved for clinical and commercial grade production. The lease terminates in 2017. In April 2014 we entered into a lease with the AMC for an additional office facility of approximately 7,100 square feet, also located at the AMC campus. The lease terminates in 2017. We have also leased a facility in Lexington, Massachusetts, where we are close to completing the build out and qualification of a manufacturing facility of approximately 53,000 square feet. 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. We believe that our existing facilities are adequate to meet

current needs and that suitable alternative spaces will be available in the future on commercially reasonable terms. See "Operating and Financial Review and Prospects—Liquidity and Capital Resources—Capital Expenditures" and "—Contractual Obligations and Commitments."

#### **ITEM 4A: UNRESOLVED STAFF COMMENTS**

Not applicable.

#### **ITEM 5: OPERATING AND FINANCIAL REVIEW AND PROSPECTS**

The following is a discussion of our financial condition as of December 31, 2014 and 2013 and results of operations and cash flows for the twelve months ended December 31, 2012, 2013 and 2014. You should read the following discussion and analysis of our financial condition and results of operations in conjunction with the "Key Information—Selected Financial Data" section of this annual report and our consolidated financial statements and related notes appearing elsewhere in this annual report. In addition to historical information, this discussion contains forward-looking statements based on our current expectations that involve risks, uncertainties and assumptions. Our actual results may differ materially from those anticipated in these forward-looking statements as a result of various factors, including those set forth in the "Risk Factors" and "Forward-Looking Statements" sections and elsewhere in this annual report.

##### **Overview**

uniQure is a leader in the field of gene therapy and has a technology platform that we use as the basis for our proprietary and collaborative product lines across multiple therapeutic areas. Our core gene therapies include AMT-060 for the treatment of hemophilia B, in which we initiated a Phase I/II clinical trial in the first quarter of 2015; our preclinical S100A1 therapeutic for the treatment of congestive heart failure, and Glybera the first and currently the only gene therapy product to receive regulatory approval in the European Union.

We seek to develop gene therapies targeting a range of liver-based, cardio/metabolic and CNS indications, from ultra-orphan diseases, such as LPLD (for which Glybera is designated), to orphan diseases such as hemophilia B and Sanfilippo B syndrome, to common diseases that affect far larger populations, such as congestive heart failure and Parkinson's disease. The core of our approach is our modular technology backbone, which allows us to advance our programs in multiple therapeutic areas using validated components of our technology and safety and efficacy data from earlier clinical studies, in multiple therapeutic areas with the potential to reduce development risk, cost and time to market. As part of our strategy, we are accessing important medical expertise for our therapeutic focuses through strong ties with academic thought leaders and clinical institutions. For cardio/metabolic diseases we are building a center of expertise in our German subsidiary, uniQure GmbH, in close cooperation with leading academic clinicians and surgeons at the university hospital and heart center in Heidelberg, Germany. Our CNS activities are based on strong collaborations with the University of California at San Francisco, the National Institutes of Health, and the Institut Pasteur, Paris, France. Our hemophilia B product originates from St. Jude Children's research Hospital in Memphis, Tennessee. We also seek to collaborate with or acquire emerging companies within our chosen therapeutic areas that are conducting or sponsoring early-stage clinical trials. Our collaborations allow us to cost-effectively obtain access to pre-clinical and early-stage programs without expending significant resources of our own. We generally have the rights to the data generated in these collaborator-sponsored programs, but do not control their design or timing. Our collaboration programs include gene therapy candidates for Parkinson's disease, Sanfilippo B syndrome, Acute Intermittent Porphyria and amyotrophic lateral sclerosis.

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. On February 5, 2014, we successfully completed our initial public offering, placing 5,400,000 shares at \$17 per share, raising total gross proceeds of \$91.8 million (€67.3 million) and net proceeds of \$85.4 million (€62.6 million) after commissions but before expenses. From our first institutional venture capital financing in 2006 until our initial public offering, we funded our operations primarily through private and public placements of equity securities, and other convertible debt securities, in the aggregate amount of €204.4 million (\$266.5 million). During this period, we also received total other income, consisting principally of government grants and subsidies, of €6.7 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 and subsequently delisted from that exchange in 2012. We acquired the business of AMT in the first half of 2012.

As of December 31, 2014, we had cash and cash equivalents of €53.2 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 €37.0 million in 2014 and €26.8 million in 2013. As of December 31, 2014, we had an accumulated deficit of €181.1 million. We anticipate that our expenses will increase substantially in the future as we:

- Conduct a Phase I/II clinical trial of AMT-060 for hemophilia B in collaboration with Chiesi;
- to expand our research capabilities and corporate infrastructure to support our collaboration with BMS to develop gene therapies in cardiovascular and other target-specific areas;
- complete our EMA-mandated post-approval clinical trials of Glybera and maintain 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;
- 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;
- exercise our options to acquire rights and pursue development of certain product candidates, the development of which is currently being conducted and funded by third parties;
- acquire or in-license rights to new therapeutic targets or product candidates;
- enter into collaboration agreements with third parties to collaborate on the research and development of potential product candidates;
- establish a sales, marketing and medical affairs infrastructure in the United States;
- fund the ongoing operations of our new manufacturing facility in Lexington, Massachusetts;
- fund expenses in connection with our 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; and
- add operational, financial and management information systems and related finance and compliance personnel.

## **Collaboration and License 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. 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 later 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 €5.8 million as of December 31, 2014, until the earlier of repayment in full of such amount and 2019.

### *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.

#### ***Strategic Collaboration: 4D Molecular Therapeutics***

In January 2014, we entered into a collaboration and license agreement with 4D Molecular Therapeutics, or 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, has established a laboratory, which we are funding at a cost of approximately \$3.0 million in aggregate through 2016, 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 609,744 ordinary shares in connection with this collaboration, and will recognize resulting share-based payment expense through 2016. 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.

#### ***InoCard Acquisition***

In July 2014, we acquired InoCard GmbH, an early-stage biotechnology company focused on the development of gene therapy approaches for cardiac disease. InoCard was founded in December 2013 as a spin-off of the University of Heidelberg. InoCard has developed a novel gene therapy through preclinical proof of concept for the one-time treatment of congestive heart failure (CHF). InoCard founders Prof. Patrick Most and Prof. Hugo Katus have joined uniQure as Managing Director of uniQure in Germany and Chairman of the Scientific Advisory Board, for Cardiovascular Diseases, respectively.

Under the terms of the agreement, the Company paid the InoCard shareholders an upfront payment of approximately €3,000,000 (€1,500,000 in cash and €1,500,000 in uniQure shares), and will receive a further €14,500,000 in success-based milestone payments upon achieving certain clinical and regulatory targets. Upon a successful commercial launch of a developed product, the sellers will further receive a royalty payment of 0.5% of the net product sales. The €14,500,000 in milestones is payable, at the Company's sole discretion, in either cash or a variable number of uniQure shares, based on the then current share price.

#### ***Other 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.



## 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 twelve months ended December 31, 2014, we recognized collaboration revenues of €3.8 million associated with 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 twelve months ended December 31, 2014, we also recognized license revenues of €0.9 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 €15.6 million of these license revenues deferred as of December 31, 2014, 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 revenue 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 "Cost of Goods Sold" below. Accordingly, we do not currently expect that we will be required to repay any of these grants.

In 2014 uniQure, Inc., our wholly owned subsidiary, received a grant from Massachusetts Life Science Company, or MLSC, under its Job Incentive Program (New Job Creation). Under our agreement with MLSC, we committed to the creation of 50 new permanent, full-time equivalent, employment roles for 2014. uniQure, Inc. received the cash award of \$917,000 from MLSC in October 2014. The MLSC grant is a five-year contract, and uniQure, Inc. has decided to amortize the grant monthly, over the five-year life. Although, the grant was received in October, uniQure, Inc. did not reach the minimum number of full-time equivalent roles required under the agreement until December 2014. Therefore, the amortization of the grant started in December 2014.

### ***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 December 31, 2014, the total amount of principal and interest outstanding was €5.8 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. In line with the timing of recording our product revenue 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, 2019. Amounts that remain outstanding as of December 31, 2019, 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 cost of goods sold for sales of Glybera in the United States would be significantly lower than our cost of goods sold for sales of Glybera in the European Union due principally to the existence of lower royalty obligations on U.S. sales.

## **Research and Development Expenses**

Research and development expenses consist principally of expenses associated with employees, manufacturing facilities, clinical development, collaborations 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 December 31, 2014, we incurred an aggregate of €130.4 million in research and development expenses. In addition, we began to capitalize our development expenses related to Glybera from March 21, 2013. We capitalized €3.7 million of such expenses in 2014, 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:

- *Hemophilia B.* We have initiated a Phase I/II clinical trial of AMT-060 for the treatment of hemophilia B in the first quarter of 2015 in collaboration with Chiesi. Under our co-development agreement, we and Chiesi will each bear half of the development costs of this program.
- *Congestive heart failure.* In the third quarter of 2014, we started to incur costs related to the pre-clinical development of product candidates targeting the S100A1 gene, the rights to which we obtained through our acquisition of InoCard in July 2014.
- *Glybera.* We are undertaking preparations for the EMA-mandated post-approval clinical trials and patient registry and preparing for the initiation of that study under an IND with the FDA in the first half of 2016. 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.
- *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 collaborator, 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 twelve-month periods ended December 31, 2013 and 2014:

(€ in thousands, except percentages)	Twelve months ended December 31,				
	2013	%	2014	%	Change
					%
Glybera program*	2,727	20.7	7,051	20.8	0.1.
Hemophilia B program	3,034	23.0	6,557	19.3	(3.7)
AIP program	241	1.8	229	0.7	(1.1)
CNS programs	822	6.2	306	0.9	(5.3)
Technology platform development and research programs	6,358	48.3	19,789	58.3	10.0
Total	13,182	100	33,932	100	—

\* 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.

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 trials and additional development work to seek FDA approval, of approximately €7.0 million; in addition, we will incur significant related employee expenses.

In addition, in connection with our collaboration and license agreement with 4D Molecular Therapeutics, we are incurring expenses to fund a joint research effort with 4D. Further, we granted options to purchase an aggregate of 609,744 of our 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 three year future service period, and will have a significant impact on our expenses recognized. Finally, to the extent certain pre-clinical, clinical and regulatory milestones are met, we will make milestone payments to 4D.

### ***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 in our commercial, finance and compliance groups, and as we commence manufacturing operations in our facility in Lexington, Massachusetts. We also 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 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 Gains / Losses—Net***

Other gains / losses—net consist 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.

Over time we have entered into various financing arrangements with our investors, including convertible notes issued in 2009 and converted into ordinary shares in April 2012, and further convertible notes issued in 2012 and 2013, which were converted into ordinary shares in July 2013. The notes that were converted in 2013 have a surviving embedded 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 expenses in 2013 consisted 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. In 2014, finance expenses are driven by the interest due on the Hercules venture debt loan, which was amended and increased in June 2014 to a total loan amount of \$20.0 million.

## Results of Operations

### Comparison of the twelve months ended December 31, 2013 and 2014

(€ in thousands, except percentages)	Twelve months ended December 31,			% Change 2013 to 2014 %
	2012	2013	2014	
<b>Revenues:</b>				
License revenues	—	440	883	101
Collaboration revenues	—	2,503	3,802	52
Total revenues	—	2,943	4,685	59
Cost of goods sold	—	(800)	—	—
Other income	649	585	773	32
<b>Expenses:</b>				
Research and development expenses	(10,231)	(13,182)	(33,932)	157
Selling, general and administrative expenses	(4,564)	(11,628)	(11,167)	(4)
Other gains / losses, net	(45)	(453)	5,807	—
Operating result	(14,191)	(22,535)	(33,834)	50
Finance income	22	102	254	149
Finance expense	(547)	(4,387)	(3,460)	(21)
Net loss	(14,716)	(26,820)	(37,040)	38

## Revenues

License revenues for the twelve months ended December 31, 2014 were €0.9 million, a 101% increase from the €0.4 million for the twelve months ended December 31, 2013. The Chiesi license payment was received in July 2013, thus the twelve-month period ending December 31, 2013 only includes a partial period of amortization, whereas the current year reflects a full period of amortization. We recorded no licence revenue for the twelve months ended December 31, 2012.

Collaboration revenues for the twelve months ended December 31, 2014 were €3.8 million, a 52% increase from the €2.5 million for the twelve months ended December 31, 2013. This increase reflects increased reimbursement from Chiesi of development activities in connection with our hemophilia B program. We recorded no collaboration revenue for the twelve months ended December 31, 2012.

## Cost of Goods Sold

Cost of goods sold of €0.8 million in the twelve months ended December 31, 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 twelve months of 2012 and 2014.

**Other Income**

Other income for the twelve months ended December 31, 2014 was €0.77 million, a 32% increase from the €0.59 million recognized for the twelve months ended December 31, 2013. This change reflects a slight increase in employee-related government grants received.

**Research and Development Expenses**

Research and development expenses for the twelve months ended December 31, 2014 were €33.9 million, a 157% increase from the €13.2 million incurred for the twelve months ended December 31, 2013. This increase reflected the expansion of our research and development activities to support our hemophilia B program and our other product candidates and research programs, as well as the build-up of staff in our Lexington facility. In addition, as part of our strategic collaboration with 4D Molecular Therapeutics entered into in January 2014, we incurred increased research and development expenses related to certain share-based payments of €6.3 million in relation to the management of 4D Molecular Therapeutics.

Glybera-related raw materials that cannot be used for commercial purposes are accounted for as research and development expenses; Glybera-related materials, including raw materials 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.

The amount presented for the twelve months ended December 31, 2012 is reflecting the strategic reorganization and its related reduction in workforce and overall level of activity.

**Selling, General and Administrative Expenses**

Selling, general and administrative expenses for the twelve months ended December 31, 2014 were €11.2 million, a 4% decrease from the €11.6 million incurred for the twelve months ended December 31, 2013. This decrease resulted principally from higher legal and audit related expenses incurred in 2013 associated with the preparation of our initial public offering, partially offset by an increase in expenses related to being a public company, and the continued build-out of the administrative functions. The amount presented for the twelve month period ended December 31, 2012 is largely reflective of the costs associated with the corporate restructuring in addition to the expenses associated with the regular administrative functions.

**Other losses—Net**

Other gains / losses—net for the twelve months ended December 31, 2014 were a gain of €5.8 million, compared with a loss of €0.45 million for the twelve months ended December 31, 2013. This increase reflects changes in the foreign exchange rate between the euro and the U.S. dollar. We have not established any formal practice to manage the foreign exchange risk against our functional currency. Other losses were not material for the twelve month period ended December 31, 2012.

**Finance Income**

Finance income for the twelve months ended December 31, 2014 was €0.254 million, a 149% increase from the €0.1 million for the twelve months ended December 31, 2013. This reflects the increased interest income associated with our higher average cash balance in 2014 compared to 2013. Finance Income was not material for the twelve month period ended December 31, 2012.

**Finance Expense**

Finance expense for the twelve months ended December 31, 2014 was €3.5 million, a 21% decrease from the €4.4 million for the twelve months ended December 31, 2013. The amount in 2013

primarily related to the revaluation of the embedded derivatives upon the conversion of the loan, which totaled €3.5 million, in addition to the interest on the Hercules venture debt loan received in June 2013. The €3.5 million in 2014 related to the interest on the Hercules venture debt loan for a total of €1.7 million combined with a foreign exchange result on the loan of €1.8 million. Finance expense for the twelve month period ended December 31, 2012 is reflective of the interest on the convertible loan and the charge on the movement in the value of the derivative element of the convertible loan, converted following the reorganization 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. From our first institutional venture capital financing in 2006 until our initial public offering in February 2014, we funded our operations primarily through private and public placements of equity securities, and convertible and other debt securities, in the aggregate amount of €204.4 million (\$266.5 million). During this same period, we also received total other income, consisting principally of government grants and subsidies, of €6.7 million, and total nonrefundable collaboration funding of €17.0 million, and \$20.0 million (€14.7 million) in venture debt financing.

We had a net loss of €37.0 million in 2014, €26.8 million in 2013 and €14.7 million in 2012. As of December 31, 2014, we had an accumulated deficit of €181.0 million.

## Cash flows

Our cash and cash equivalents as of December 31, 2014 were €53.2 million. The table below summarizes our consolidated cash flow data for the unaudited twelve-month periods ended December 31, 2012, 2013 and 2014:

(€ in thousands)	Twelve months ended December 31,		
	2012	2013	2014
Cash (used in) / generated by operating activities	(11,277)	(4,136)	(25,425)
Cash used in investing activities	(832)	(5,971)	(20,451)
Cash provided by financing activities	11,272	33,642	69,457

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

Net cash used in operating activities was €25.4 million in the twelve months ended December 31, 2014; a 515% increase compared to the net cash used by operating activities of €4.1 million in the twelve months ended December 31, 2013. The amount for 2013 reflected the receipt of the upfront payment under our collaboration agreements with Chiesi. Net cash used in operating activities in the twelve months ended December 2012 was the result of normal operations with a reduced workforce and activity following the corporate reorganization undertaken in 2011 and 2012.

In 2014 we have seen continued expenditures in progressing our pipeline products as well as the expense associated with the start-up of the operations in our Lexington, Massachusetts manufacturing facility.

### Net Cash Used in Investing Activities

Net cash used in investing activities was €20.5 million in the twelve months ended December 31, 2014, a 243% increase from €6.0 million in the twelve months ended December 31, 2013. The increase primarily reflected the continued build-out of our manufacturing facility in Lexington, Massachusetts.



Net cash used in investing activities in the twelve months ended December 31, 2012 related mostly 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 €69.5 million in the twelve months ended December 31, 2014, a 106% increase from €33.6 million in the twelve months ended December 31, 2013. The increase primarily reflected the receipt of net proceeds of €62.0 million in connection with our initial public offering in February 2014, and the amendment to the Hercules venture debt loan that resulted in a cash inflow of a net €7.2 million. Net cash generated from financing activities for the twelve months ended December 31, 2012 reflected our private placements of convertible notes and equity securities in connection with and following our corporate reorganization.

### *Cash and Funding Sources*

The table below summarizes our sources of financing for the twelve months ended December 31, 2012, 2013 and 2014.

(€ in thousands)	Equity Capital(1)	Convertible Notes	Other Debt	Total
twelve months ended December 31, 2014	62,429	—	7,184	69,613
twelve months ended December 31, 2013	14,294	11,999	7,492	33,785
twelve months ended December 31, 2012	9,774	1,498	—	11,272
Total	86,497	13,497	14,676	114,670

(1) Excludes shares issued upon conversion of convertible notes

Our sources of financing in the twelve months ended December 31, 2014 were:

- the issuance and sale of 5,400,000 ordinary shares at an initial public offering price of \$17.00 per share, with net proceeds of €62.0 million, after commissions and expenses;
- an additional venture loan in the principal amount of \$10.0 million from Hercules Technology Growth Capital; and
- as of December 31, 2014, we had debt of €16.4 million, which consisted solely of amounts outstanding under the Hercules Agreement.

Sources of financing for the twelve month periods ended December, 31 2012 and 2013 were the issuance and sales of our convertible notes, the initial venture debt loan with Hercules Technology Growth Capital, the issuance and sale of ordinary shares to Chiesi, and the issuance and sale of shares to employees and existing shareholders.

### *Funding Requirements*

We believe our cash and cash equivalents as at December 31, 2014, when taken together with additional funds raised since that date following the collaboration with Bristol-Myers Squibb, will enable us to fund our operating expenses, including our debt repayment obligations as they become due, and capital expenditure requirements, for the next twelve months and beyond. For further disclosure please refer to Item 4 for a description of the BMS Collaboration. 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 potential to receive future consideration pursuant to our collaboration with BMS, which is largely contingent on achieving certain research, development, regulatory and sales milestones;
- 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, our ability to obtain research and development funding and achieve milestones under these agreements and our ability to enter into other such new arrangements in the future;
- the progress and results of our current and planned clinical trials, including for Glybera and AMT-060 for hemophilia B, as well as those of our collaborators;
- the scope, progress, results and costs of preclinical development, laboratory testing and clinical trials for S100A1 in congestive heart failure and 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 repayments of the principal amount of our venture debt loan with Hercules Technology Growth Capital, that contractually will start in January 2016 and will run through June 2018;
- the extent to which we acquire or in-license other products or technologies; and
- the costs associated with maintaining quality compliance and optimizing our manufacturing processes, including the operating costs associated with our Lexington, Massachusetts manufacturing facility.

We have no committed sources of additional financing, other than our collaboration agreements with Chiesi and, upon closing, our collaboration with BMS. 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. 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. To the extent we need to finance our cash needs through equity offerings or debt financings, such financing may be subject to unfavorable terms including without limitation, the negotiation and execution of definitive documentation, as well as credit and debt market conditions, and we may not be able to obtain such financing on terms acceptable to us or at all. If financing is not available when needed, including through debt or equity financings, or is available only on unfavorable terms, we may be unable to meet our cash needs. 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, which could have a material adverse effect on our business, financial conditions, results of operations and cash flows. No assurances can be given that the company's actions as described above will ultimately be successful in meeting its funding needs. Please refer to the Risk Factors section, and in particular the Risk Factor entitled: "*We will likely need to raise additional funding, which may not be available on acceptable terms, or not at all. Failure to obtain capital when needed may force us to delay, limit or terminate our product development efforts or other operations which could have a material adverse effect on our business, financial conditions, results of operations and cash flows*" for further information.

### Capital Expenditures

The following table sets forth our capital expenditures for the twelve months ended December 31, 2012, 2013 and 2014:

(€ in thousands)	Twelve months ended December 31,		
	2012	2013	2014
Investments in property, plant and equipment	(392)	(1,336)	(15,769)
Investments in intangible assets	(553)	(4,652)	(3,367)
Acquisition of Business	—	—	(1,463)
Total	<u>(945)</u>	<u>(5,988)</u>	<u>(20,599)</u>

In the third quarter of 2014, we completed the build-out, started in 2013, of a leased manufacturing facility in Lexington, Massachusetts of approximately 53,000 square feet. The total construction costs amount to approximately \$16.8 million (€13.8 million), of which the landlord has paid \$7.3 million (€6.0 million) in landlord improvements. In addition, we anticipate the total investment in property, plant and equipment to be approximately \$8.2 million (€6.7 million). As of December 31, 2014, we had capitalized \$22.5 million (€18.5 million) and had contractual commitments of a further \$1.5 million (€1.2 million) related to the further build-out of the facility. In addition, we provided a landlord deposit of \$1.2 million (€1.0 million).

The investments in Intangible Assets relate to the capitalization of licenses and the ongoing capitalization of Glybera-related development costs.

The Acquisition of Business line item relates to the InoCard transaction that was complete on July 31, 2014.

## Contractual Obligations and Commitments

The table below sets forth our contractual obligations and commercial commitments as of December 31, 2014 that are expected to have an impact on liquidity and cash flows in future periods.

(€ in thousands)	Payments due by period				
	Less than 1 year	Between 1 and 2 years	Between 2 and 5 years	More than 5 years	Total
License maintenance obligations(1)(2)	306	—	—	—	306
Debt obligations(3)	1,710	7,773	11,480	—	20,963
Operating lease obligations	1,918	1,830	4,564	7,285	15,597
Finance lease obligations	168	134	—	—	302
Construction commitment US Facility	1,200	—	—	—	1,200
Total	5,302	9,737	16,044	7,285	38,368

- (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 included within the research commitments detailed in the notes to the financial statements.
- (3) Amounts disclosed includes both interest expense and principal repayments.

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, in either milestones or royalties, may be material.
- Our obligations to repay the Dutch technical development loan described below.
- 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.
- Obligations to the former shareholders of InoCard. These milestone obligations can be settled in either cash or ordinary shares.

### Hercules Loan and Security Agreements

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 (€7.4 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%.

On June 26, 2014, we entered into an amended and restated Loan and Security agreement (which amends and replaces the original Loan Agreement) which increased the aggregate amount that we may borrow up to \$20,000,000 (€14,600,000), net of expenses for facility charges of 1.00% plus expenses related to legal counsel. The additional amount of \$10,000,000 (€7,344,000) was received net of expenses of \$218,000 (€160,000). This resulted in a total cash inflow of \$9,782,000 (€7,184,000). The new loan commitment is \$20,000,000 with an interest rate of 10.25% which matures over a period of 48 months. Also included are two back-end fees of \$345,000 and \$250,000, due October 2016 and June 2018 respectively. The interest-only period is 18 months. We are required to repay the loan in monthly

principal installments from January 2016 through June 2018. As the terms of the amended loan agreement changed significantly compared to the original loan agreement (maturity date, interest rate, payback schedule), we fully amortized the unamortized transaction costs at issue, which is required under IAS39, resulting in an extra amortization charge through profit and loss in 2014 of \$193,000 (€141,000).

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 37,174 warrants, at an exercise price of \$13.45 per share. The warrant included in the Loan and Security Agreement is not closely related to the host contract and therefore has been split and accounted-for separately as a financial derivative measured at fair value through profit or loss. The fair value of this derivative as of December 31, 2014 was €207,000 compared to €217,000 on December 31, 2013.

During the quarter ended September 30, 2014, the current obligation of this loan facility reduced to nil, as the amended agreement introduced a further extension of the interest only period to January 1, 2016.

The borrowings under the Loan and Security Agreement were classified as non-current borrowings of €16.4 million, net of expenses, as of December 31, 2014. For the twelve-month period ended December 31, 2014, we recorded €1.6 million as finance expenses in relation to the Amended Loan and Security Agreement, compared to €0.5 million for the same period in 2013, and noted the loan commences in the third quarter of 2013.

The exchange result on the borrowings under the Loan and Security Agreement amounts to €1.8 million.

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. This restriction on the cash reserves only relates to the location of the cash reserves, but all cash reserves are at free disposal of the Company. The Loan and Security Agreement contains default provisions that include the occurrence of a material adverse effect, as defined therein, which would entitle Hercules to declare all principal, interest and other amounts owed by us immediately due and payable. As of December 31, 2014, we were in compliance with these covenants in all material respects.

## **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. Our loan from Hercules, was received and is repayable in U.S. dollars, and starting in the fourth quarter of 2013 and throughout

2014, we incurred obligations in U.S. dollars in respect of our manufacturing facility in Lexington, Massachusetts, as described above.

Our interest rate risk arises from short and long term borrowings. 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, 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%. On June 26, 2014, we entered into an amended and restated loan agreement with Hercules, which replaces the original loan agreement. Pursuant to the amended and restated agreement, the total loan commitment is now \$20,000,000 with an interest rate of 10.25% which matures over a period of 48 months and two back-end fees of \$345,000 and \$250,000 respectively payable in October 2016 and June 2018. The interest-only period is 18 months.

### ***Credit Risk***

Credit risk is managed on company basis. Credit risk arises from cash and cash equivalents, derivative financial instruments and deposits with banks and financial institutions, as well as credit exposures to wholesale customers, including outstanding receivables and committed transactions. We currently have no wholesale debtors other than Chiesi.

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.

### ***Liquidity Risk***

We believe that our existing cash and cash equivalents, including proceeds of our initial public offering in February 2014, the amended Hercules loan, anticipated payments under our agreements with Chiesi and the agreement entered into with Bristol-Myers Squibb will enable us to fund our operating expenses and capital expenditure requirements for at least the next 12 months.

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

Our operating and financial review and prospects 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 annual report. We believe that the following accounting policies involve the most significant judgments and estimates by management and are the most critical to fully understanding and evaluating our financial condition and results of operations.

### ***Revenue Recognition***

We did not generate any revenues from royalties or product sales for 2013 or 2014.

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, the United States and Germany. 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 annual report, since we are in a loss making position and have a history of losses. As of December 31, 2014, the total amount of tax losses carried forward under the Dutch tax regime was €145.1 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 annual report. Tax losses in the Netherlands may be carried forward for nine years.

Following the InoCard acquisition and in line with the Purchase Price Allocation under IFRS 3 we have recognized a deferred tax liability, based on the acquired asset labeled as In Process Research and Development times the German Corporate tax rate

### ***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 year ended December 31, 2014, management reviewed the carrying amount of these assets and determined that no adjustments to carrying values were required.

On assets that are not subject to amortization, we annually perform an impairment review based on the fair value less cost of disposal method. For the purpose of assessing impairment, we group assets at the lowest levels for which there are separately identifiable cash flows (cash generating units).



We currently use all material assets in the development of certain gene therapy products. Therefore, the management regularly reviews all our activities 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 revenue will depend on the success of Chiesi's commercialization efforts and our success in obtaining marketing authorization for Glybera and any other product candidates in additional countries. 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.

Next to Glybera-related intangible assets, in the impairment review we also considered the intangible assets associated with other pipeline products and the manufacturing platform. New to the review in 2014 were the In Process Research and Development and Goodwill as they arose from the InoCard acquisition.

We have determined that no impairment charge is required for the year ended December 31, 2014. Performing a further sensitivity analysis on the fair value calculation (by for example, reducing the fair value per ordinary share by 20%, as used in the calculation of the enterprise value), did not change management's conclusion that no impairment charge was required.

### ***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 2014, 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.

### **Fair value of Financial instruments**

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 Company has determined that the contingent consideration arising from the InoCard acquisition is classified as level 3 in the fair value measurement hierarchy and that the warrants after the IPO in February 2014 were to be classified as a level 2, as our shares are currently traded on NASDAQ under the symbol "QURE" and the valuation of the warrants is derived from the quoted share price. Please refer to note 4 to our consolidated financial statements contained in this annual report for the sensitivity analysis relating to the fair value of the warrants and contingent consideration.

### ***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 have estimated based on a weighted average expected option life for the entire participant group;
- 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.

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 13 to our audited consolidated financial statements included elsewhere in this annual report 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 December 31, 2014, 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:

<u>GRANT DATE</u>	<u>NUMBER OF ORDINARY SHARES UNDERLYING OPTIONS GRANTED</u>	<u>EXERCISE PRICE PER ORDINARY SHARE</u>	<u>ESTIMATED FAIR VALUE PER ORDINARY SHARE AT GRANT DATE</u>	<u>RETROSPECTIVE FAIR VALUE PER ORDINARY SHARE AS OF GRANT DATE(1)</u>	<u>ESTIMATED FAIR VALUE PER OPTION AS OF GRANT DATE</u>
April 5, 2012	1,366,304	€ 3.07	€ 3.07	€ 3.07	€ 2.05
June 12, 2012	15,000	3.07	3.07	3.07	2.05
December 1, 2012	140,652	3.07	3.07	4.85	3.35
December 22, 2012	84,391	3.07	3.07	5.10	3.60
January 1, 2013	112,000	5.00	5.00	5.45	3.40
March 26, 2013	14,065	5.00	5.00	7.65	5.30
June 5/6, 2013	28,000	10.10	10.10	12.60	8.15
September 1, 2013	140,652	10.10	13.30	N/A	8.85
October 1, 2013	6,751	3.07	13.40	N/A	12.35
January 17, 2014	609,744	0.05	12.60(2)	N/A	12.55
May 27, 2014(3)	926,000	\$ 9.35	\$ 8.66(4)	N/A	\$ 5.24
October 15 2014(3)	189,000	\$ 9.63	\$ 9.63(4)	N/A	\$ 5.93

- (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.
- (2) The Euro equivalent of the initial public offering price on February 10, 2014.
- (3) The 2014 option grants after the IPO are all denominated in USD
- (4) For the 2014 option grants after the IPO the NASDAQ close on day of grant is presented

As of December 31, 2014 a total of 3,053,840 options were outstanding under (with exercise prices both in U.S. dollars and euros and ranging from €0.05 - €10.10 and \$9.35 - \$9.63) the above mentioned

grant dates, of which 1,423,175 had vested. As of December 31, 2014, the unrecognized expense related to the options which have been granted and remained outstanding was €4.8 million.

## **Recent Accounting Pronouncements**

There are no IFRS standards as issued by the IASB or interpretations issued by the IFRS interpretations committee that are effective for the first time for the financial year beginning on or after January 1, 2014 that had or are expected to have a material impact on our financial position.

A number of new standards and amendments to standards and interpretations (e.g IFRS9, IFRS15) are effective for annual periods beginning after January 1, 2014 and have not been applied in preparing these consolidated financial statements.

We are yet to assess the full impact of the above standards but none of these are expected to have a material effect on our consolidated financial statements.

## **C. Research and Development Expenses, Patents and Licenses, etc.**

See "Information on the Company—Business Overview—Intellectual Property" and "Operating and Financial Review and Prospects."

## **D. Trend Information**

See "Operating and Financial Review and Prospects."

## **E. 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, 2019, 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, 2019, or if there are no revenues generated from Glybera, the remaining balance will be forgiven. The amount of this contingent commitment as of December 31, 2014 totaled €5.8 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 hereof, and during the periods presented herein, we did not have any other off-balance sheet arrangements.

## F. Tabular Disclosure of Contractual Obligations

### Contractual Obligations and Commitments

The table below sets forth our contractual obligations and commercial commitments as of December 31, 2014 that are expected to have an impact on liquidity and cash flows in future periods.

(€ in thousands)	PAYMENTS DUE BY PERIOD				TOTAL
	LESS THAN 1 YEAR	BETWEEN 1 AND 2 YEARS	BETWEEN 2 AND 5 YEARS	MORE THAN 5 YEARS	
License maintenance obligations(1)(2)	306	—	—	—	306
Debt obligations	1,710	7,773	11,480	—	20,963
Operating lease obligations	1,918	1,830	4,564	7,285	15,597
Finance lease obligations	168	134	—	—	302
Construction commitment US Facility	1,200	—	—	—	1,200
Total	5,302	9,737	16,044	7,285	38,368

- (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 included within the research commitments detailed 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, in either milestones or royalties, may be material.
- Our obligations to repay the Dutch technical development loan described below.
- 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.
- Obligations to the former shareholders of InoCard. These milestone obligations can be settled in either cash or ordinary shares.

## G. Safe harbor

See "Forward-Looking Statements".

**ITEM 6: DIRECTORS, SENIOR MANAGEMENT AND EMPLOYEES****A. Directors and Senior Management**

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.

**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 annual report. 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 are independent under applicable NASDAQ standards:

<u>NAME</u>	<u>AGE</u>	<u>POSITION</u>	<u>MEMBER SINCE(1)</u>	<u>TERM EXPIRES</u>
Ferdinand Verdonck	72	Member of the Supervisory Board (Chairman)	2012	2017
Joseph M. Feczko	66	Member of the Supervisory Board	2012	2016
Will Lewis	46	Member of the Supervisory Board	2014	2017
David Schaffer	45	Member of the Supervisory Board	2014	2016
Sander Slootweg	46	Member of the Supervisory Board	2012	2015
Paula Soteropoulos	47	Member of the Supervisory Board	2013	2017
Sander van Deventer	60	Member of the Supervisory Board	2012	2016

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

**Ferdinand Verdonck** has served as our chairman since July 2012 and served as chairman of the AMT supervisory board from April 2007 until July 2012. He is a director on the boards of J.P. Morgan European Investment Trust, Groupe SNEF, Laco Information Services and Virtus Funds. From 1992 to 2003, he was the managing director of Almanij NV, a financial services company which has since merged with KBC, and his responsibilities included company strategy, financial control, supervision of executive management and corporate governance, including board participation in publicly-traded and privately-held companies in many countries. He served as a member of the board of directors and chairman of the audit committee of two biotechnology companies in Belgium, Movetis and Galapagos. He has previously served as chairman of Banco Urquijo, a director of Dictaphone Corporation and a director of the Dutch Chamber of Commerce for Belgium and Luxembourg, member of the General Council and chairman of the audit committee of the Vlerick Leuven Ghent Management School. Mr. Verdonck holds a law degree from KU Leuven and degrees in economics from KU Leuven and the University of Chicago. We believe that Mr. Verdonck is qualified to serve on our supervisory board due to his expertise in the financial services and manufacturing industries and his service on the boards of directors of other companies.

**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.

**Will Lewis** has served as a member of our supervisory board since June 2014. Mr. Lewis is currently President, Chief Executive Officer and member of the Board of Directors of Insmmed, a biopharmaceutical company specialized in inhalation therapies for orphan lung diseases. Prior to joining Insmmed in 2012, he was President and Chief Financial Officer of Aegerion Pharmaceuticals, Inc., which he also co-founded. At Aegerion, he played a pivotal role in re-orienting the company's strategy to focus on orphan disease indications. He previously worked in the U.S. and Europe in investment banking for JP Morgan, Robertson Stephens and Wells Fargo. During his time in banking, he was involved in a broad range of domestic and international capital raises and advisory work valued at more than \$20 billion. He serves on the Board of Directors of Oberlin College and is a member of the Visiting Committees of the Weatherhead School of Management of Case Western Reserve University and The Hawken School. He holds a B.A. from Oberlin College and an M.B.A./J.D. from Case Western Reserve University. We believe that Mr. Lewis is qualified to serve on our supervisory board due to the depth of his experience in the biotechnology and finance industries.

**David Schaffer** has served as a member of our supervisory board since January 2014. 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.

**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.

**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.

**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 February 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.

### **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 annual report. The business address of our management board members is our registered office address at Meibergdreef 61, Amsterdam 1105 BA, the Netherlands.

<u>NAME</u>	<u>AGE</u>	<u>POSITION</u>	<u>DATE OF APPOINTMENT</u>
Jörn Aldag	55	Chief Executive Officer	October 4, 2009
Matthew Kapusta(1)	42	Chief Financial Officer	January 1, 2015

- (1) Matthew Kapusta was appointed Chief Financial Officer in January 2015. His appointment to the management board is subject to approval at our 2015 annual general meeting.

**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.

**Matthew Kapusta** has served as our chief financial officer since January 2015. Mr Kapusta has nearly 20 years of experience in the life sciences industry. Most recently, Mr. Kapusta was Senior Vice President at AngioDynamics responsible for corporate development, strategic planning and national accounts. Prior to AngioDynamics, he served as Vice President, Finance for Smith & Nephew Orthopaedics. Mr. Kapusta's career also includes more than a decade of investment banking experience focused on emerging life sciences companies. Mr. Kapusta was Managing Director, Healthcare Investment Banking at Collins Stewart, and held various positions at Wells Fargo Securities, Robertson Stephens and PaineWebber. Mr. Kapusta holds a Master of Business Administration from New York University's Stern School of Business, a Bachelor of Business Administration from University of Michigan's Ross School of Business and earned his Certified Public Accountant license in 1996 while at Ernst & Young. Mr. Kapusta's appointment to our management board is subject to approval at our 2015 annual general meeting. We believe that Mr. Kapusta is qualified to serve on our management board due to his broad expertise in the biotechnology and finance industries.

### 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 annual report. The business address of the members of our senior management is our registered office address at Meibergdreef 61, Amsterdam 1105 BA, the Netherlands.

<u>NAME</u>	<u>AGE</u>	<u>POSITION</u>
Eric Goossens	49	Chief Operating Officer
Christian Meyer, M.D.	47	Chief Medical Officer
Harald Petry	55	Chief Science Officer
Hans Preusting	52	Chief Business Officer
Hans Christian Rohde	57	Chief Commercial Officer

**Eric Goossens** has served as our chief operating officer since 2014, prior to which he worked for Dätwyler Pharma Packaging Belgium N.V. for three years as Site Director responsible for the Belgium and Germany sites. Before this, he held leadership positions in operations at Sekisui S-LEC Europe B.V., where he was responsible for the Film Plant Operations and Supply Chain. From 2002 to 2006 he was Project Manager and Director Production Operations at Centocor B.V. where he played an important role in a major site expansion, building a new biopharmaceutical production facility. Mr. Goossens holds a Master's degree in Chemistry from the University of Utrecht, the Netherlands, as well as a Master of Engineering from the University of Twente, the Netherlands. His educational background includes international executive leadership programs at the business schools of INSEAD and IMD.

**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 master of science from the University of Copenhagen and a master of business administration from the Birmingham Business School, the University of Birmingham in the United Kingdom.

## B. Compensation

The below table sets out a breakdown of the compensation, in aggregate, for members of our supervisory board, management board and senior management:

		SHORT TERM EMPLOYEE BENEFITS	SHARE- BASED PAYMENTS(1)	POST- EMPLOYMENT BENEFITS	ADVISORS FEES	TERMINATION BENEFITS	TOTAL
		(€ in thousands)					
Year ended December 31, 2014	Supervisory Board	0	162	0	178	0	340
	Management Board	646	660	25	0	0	1,331
	Senior Management	1,791	1,437	208	0	0	3,436
		<u>2,437</u>	<u>2,259</u>	<u>233</u>	<u>178</u>	<u>0</u>	<u>5,107</u>

- (1) For information on share ownership and options held by our supervisory directors, managing directors and senior management, please see "Major Shareholders and Related Party Transactions—Major Shareholders."

For further detail on compensation of members of our supervision board, management board and senior management, see Note 31 to the audited consolidated financial statements included elsewhere in this annual report.

## **C. Board Practices**

### **Committees of the Supervisory Board**

We 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***

Our audit committee consists of Mr. Lewis (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 oversees our accounting and financial reporting processes and the audits of our consolidated financial statements. The audit committee is 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***

Our remuneration committee consists of Messrs. van Deventer (Chairman), and Verdonck. Each member satisfies the independence requirements of the NASDAQ listing standards. The remuneration committee assists 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 is 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***

Our nominating and corporate governance committee consists of Messrs. van Deventer (Chairman) and Verdonck. Each member satisfies the independence requirements of the NASDAQ listing standards. The nominating and corporate governance committee assists 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 is 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.

For information on the current term of office and the period during which the members of our supervisory board, management board and our senior management have served in office see "—Directors and Senior Management."

#### **D. Employees**

As of December 31, 2014, we had a total of 162 employees, of whom 27 had an M.D. or Ph.D. degree, or the foreign equivalent. Of these employees, 22 were engaged in research and development, three in clinical development, and two in business development functions. We also engaged 28 consultants and contract workers. We do not currently have in place a works council.

#### **E. Share Ownership**

See "Major Shareholders and Related Party Transactions."

## ITEM 7: MAJOR SHAREHOLDERS AND RELATED PARTY TRANSACTIONS

### A. Major Shareholders

The following table sets forth information with respect to the beneficial ownership of our ordinary shares as of March 31, 2015 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 "Total Percentage" is based on a total of 18,428,866 ordinary shares outstanding as of March 31, 2015, determined on the following basis: 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 March 31, 2015 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	TOTAL PERCENTAGE
<b>Major Shareholders:</b>		
Entities affiliated with Forbion(1)	4,393,524	23.8%
Cooperatieve Gilde Healthcare II U.A.(2)	1,740,315	9.4%
Coller International Partners V-A, L.P.(3)	6,512,045	35.1%
Chiesi Farmaceutici S.p.A.(4)	1,109,214	6.0%
FMR, LLC(5)	1,759,490	9.6%
<b>Management Board Members, Supervisory Board Members and Senior Management</b>		
Ferdinand Verdonck(6)	161,076	*
Sander Slootweg(7)	4,394,774	23.8%
Sander van Deventer(8)	4,404,206	23.9%
Joseph M. Feczko(9)	66,525	*
David Schaffer	152,436	*
Paula Soteropoulos(10)	6,421	*
Will Lewis (11)	2,500	*
Jörn Aldag(12)	405,079	2.2%
Matthew Kapusta(13)	0	*
Eric Goossens(14)	10,000	*
Christian Meyer(15)	92,396	*
Harald Petry(16)	148,779	*
Hans Preusting(17)	150,679	*
Hans Christian Rohde(18)	161,252	*
<b>All current management board members, supervisory board members, and senior management as a group</b>	5,762,599	29.6%
<b>Total shares held by management board members, supervisory board members, senior management and major shareholders</b>	12,490,139	63.7%

\* Represents beneficial ownership of less than one percent of our outstanding ordinary shares.

- (1) Forbion's beneficial ownership consists of (i) 987,674 ordinary shares held by Coöperatieve AAC LS U.A., or Coöperatieve; (ii) 1,520,598 ordinary shares held by Forbion Co-Investment Coöperatief U.A., or FCI; (iii) 1,865,493 ordinary shares held by Forbion Co-Investment II Coöperatief U.A., or FCI II; (iv) warrants held by FCI to purchase 9,900 ordinary shares that are exercisable as of March 31, 2015 or will become exercisable within 60 days after such date; and (v) 9,859 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. Each of Mr. Slootweg and Dr. van Deventer 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) Cooperatieve Gilde Healthcare II A.A.'s beneficial ownership consists of (i) 1,730,415 ordinary shares held by Coöperatieve Gilde Healthcare II U.A. and (ii) warrants held by Coöperatieve Gilde Healthcare II U.A. to purchase 9,900 ordinary shares that are exercisable as of March 31, 2015 or will become exercisable within 60 days after such date. The manager of Coöperatieve Gilde Healthcare II U.A. is Gilde Healthcare II Management B.V., or Gilde Management, and Gilde Management is owned by Gilde Healthcare Holding B.V., or Gilde Holding. Three managing partners, Edwin de Graaf, Marc Olivier Perret and Martenmanshurk B.V. (of which Pieter van der Meer is the owner and manager) each own 28.66% of Gilde Holding and Stichting Administratiekantoor Gilde Healthcare Holding, or Stichting, owns 14% of Gilde Holding. Stichting is controlled by Mr. de Graaf, Mr. Perret and Martenmanshurk B.V. and issued depository receipts for shares in Gilde Holding to two partners, Arthur Franken and Dirk Kersten. Each of Mr. de Graaf, Mr. Perret and Mr. van der Meer share voting and dispositive power of the shares, and disclaim beneficial ownership of the shares except to the extent of their respective pecuniary interest therein. The address of Coöperatieve Gilde Healthcare II U.A. is Newtonlaan 91, 3584 BP, Utrecht, The Netherlands.
- (3) Collier International Partners V-A, L.P.'s beneficial ownership consists of (i) 2,019,511 ordinary shares held by Collier International Partners V-A, L.P., or Collier; (ii) warrants held by Collier to purchase 99,010 ordinary shares that are exercisable as of March 31, 2015 or will become exercisable within 60 days after such date; (iii) 987,674 ordinary shares held by Coöperatieve; (iv) 1,520,598 ordinary shares held by FCI; (v) 1,865,493 ordinary shares held by FCI II; (vi) warrants held by FCI to purchase 9,900 ordinary shares that are exercisable as of March 31, 2015 or will become exercisable within 60 days after such date and (vii) 9,859 ordinary shares held by SJH. Collier is a limited partner of the Forbion funds. Collier has no dispositive or voting power over ordinary shares held by the Forbion funds and disclaims beneficial ownership of such ordinary shares except to the extent of its pecuniary interest therein. See footnote 1 above. The general partner of Collier is Collier International General Partner V, L.P. of which Collier Investment Management Limited, or CIML, is the general partner. The directors of CIML are Jeremy Joseph Collier, Cyril Joseph Mahon, Roger Alan Le Tissier, Paul McDonald, Peter Michael Hutton, John Charlton Loveless and Andrew Thane Maden Hitchon and may be deemed to share voting and dispositive power with respect to the ordinary shares held by Collier. The CIML directors disclaim beneficial ownership of such ordinary shares except to the extent of their pecuniary interest therein. The address of Collier is c/o Collier Investment Management Limited, PO Box 255, Trafalgar Court, Les Banques, St Peter Port, Guernsey, Channel Islands.
- (4) The registered office of Chiesi Farmaceutici S.p.A is Via Palermo, 26, 43122 Parma, Italy.
- (5) The registered office of FMR, LLC is 245 Summer Street, Boston, MA 02210, United States.

- (6) Mr Verdonck's beneficial ownership consists of 75,435 ordinary shares and options to purchase 85,641 ordinary shares that are exercisable as of March 31, 2015 or will become exercisable within 60 days after such date.
- (7) Mr Slootweg's beneficial ownership consists of options to purchase 1,250 ordinary shares that are exercisable as of March 31, 2015 or will become exercisable within 60 days after such date, together with securities held by funds affiliated with Forbion. See footnote 1 above.
- (8) Mr van Deventer's beneficial ownership consists of 9,432 ordinary shares and options to purchase 1,250 ordinary shares that are exercisable as of March 31, 2015 or will become exercisable within 60 days after such date, together with securities held by funds affiliated with Forbion. See footnote 1 above.
- (9) Mr Feczko's beneficial ownership consists of 27,768 ordinary shares and options to purchase 38,757 ordinary shares that are exercisable as of March 31, 2015 or will become exercisable within 60 days after such date.
- (10) Ms Soteropoulos's beneficial ownership consists of options to purchase 6,421 ordinary shares that are exercisable as of March 31, 2015 or will become exercisable within 60 days after such date.
- (11) Mr Lewis's beneficial ownership consists of options to purchase 2,500 ordinary shares that are exercisable as of March 31, 2015 or will become exercisable within 60 days after such date.
- (12) Mr Aldag's beneficial ownership consists of 39,389 ordinary shares and options to purchase 365,690 ordinary shares that are exercisable as of March 31, 2015 or will become exercisable within 60 days after such date.
- (13) Mr Kapusta's appointment to our management board is subject to approval at our 2015 annual general meeting.
- (14) Mr Goossens' beneficial ownership consists of options to purchase 10,000 ordinary shares that are exercisable as of March 31, 2015 or will become exercisable within 60 days after such date.
- (15) Mr Meyer's beneficial ownership consists of options to purchase 92,396 ordinary shares that are exercisable as of March 31, 2015 or will become exercisable within 60 days after such date.
- (16) Mr Petry's beneficial ownership consists of 627 ordinary shares and options to purchase 148,152 ordinary shares that are exercisable as of March 31, 2015 or will become exercisable within 60 days after such date.
- (17) Mr Preusting's beneficial ownership consists of 2,527 ordinary shares and options to purchase 148,152 ordinary shares that are exercisable as of March 31, 2015 or will become exercisable within 60 days after such date.
- (18) Mr Rohde's beneficial ownership consists of 13,100 ordinary shares and options to purchase 148,152 ordinary shares that are exercisable as of March 31, 2015 or will become exercisable within 60 days after such date.

#### **Holdings by U.S. Shareholders**

As of March 31, 2015, there was one holder of record of ordinary shares (Cede & Co., as nominee for DTC), holding approximately 63.8% of our ordinary shares.

#### **B. Related Party Transactions**

Since January 1, 2014, we have engaged in the following transactions with the members of our supervisory board, management board, senior management, holders of ordinary shares, and their affiliates, which we refer to as our related parties.

**2014 Initial Public Offering**

In February 2014, we completed our initial public offering, raising \$91.8 million before expenses and underwriting commissions, through an issue of our ordinary shares at a price of \$17 per share.

The following table sets forth the number of ordinary shares purchased by our related parties.

<b>SHAREHOLDER</b>	<b>NUMBER OF ORDINARY SHARES</b>
Forbion Co-Investment Coöperatief U.A.	58,823
Coller International Partners V-A, L.P.	1,029,412
Coöperatieve Gilde Healthcare II U.A.	79,412

**Grants of Options to Related Parties**

We granted options to members of the supervisory board, management board and senior management. Details of options granted are included within the beneficial ownership table above.

**4D Molecular Therapeutics Collaboration**

On January 17, 2014, we entered into a collaboration and license agreement with 4D Molecular Therapeutics, as described in "Information on the Company—Business Overview" above. 4D Molecular Therapeutics is a company co-founded by Dr. David Schaffer, who was appointed to our supervisory board on January 27, 2014 pursuant to the terms of that collaboration. In connection with this 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 304,872 ordinary shares at an exercise price of €0.05 per share to Dr. Schaffer. See "Information on the Company—Business Overview—Collaborations—Early-Stage Collaborations—4D Molecular Therapeutics."

**C. Interests of Experts and Counsel**

Not applicable.

**ITEM 8: FINANCIAL INFORMATION****A. Consolidated Statements and Other Financial Information**

See the financial statements beginning on page F-1.

**Legal Proceedings**

Except as described below, we are not involved in any material legal proceedings.

On December 11, 2013, the Company 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 the Company 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. The amount claimed is \$100,000 plus 2.5% of all proceeds, including equity investments, the Company received from Chiesi pursuant to its collaboration agreements entered into in the second quarter of 2013. The Company's engagement letter with Extera Partners contains a cap limiting the maximum liability to €5,000,000.

On May 12, 2014, the ICC appointed and confirmed a sole arbitrator. On October 1, 2014, Extera Partners LLC filed its Statement of Case which includes an estimated claim based on the formula mentioned above and on Extera's estimate of potential future revenues. A final merits hearing has been scheduled for July 2015. The Company has denied the claim and intends to vigorously defend against it. The Company filed its Statement of Defense with the International Chamber of Commerce on January 21, 2015.

**Dividends**

We do not at present plan to pay cash dividends on our ordinary shares. 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.

**B. Significant Changes**

See Note 33 to the audited consolidated financial statements included elsewhere in this annual report.

**ITEM 9: THE OFFER AND LISTING****A. Offering and Listing Details**

Not applicable.

**B. Plan of Distribution**

Not applicable.

**C. Markets**

Our ordinary shares are currently listed on The NASDAQ Global Select Market under the symbol "QURE".

The following table sets forth the high and low sale prices on The NASDAQ Global Select Market for our ordinary shares from February 5, 2014, the date of our initial public offering, through March 31, 2015.

	High	Low
<b>Annual Highs and Lows</b>		
2014 (from February 5)	\$ 18.75	\$ 8.29
<b>Quarterly Highs and Lows</b>		
First Quarter 2014	\$ 18.75	\$ 13.10
Second Quarter 2014	\$ 16.50	\$ 8.29
Third Quarter 2014	\$ 14.50	\$ 9.00
Fourth Quarter 2014	\$ 17.33	\$ 9.17
First Quarter 2015	\$ 28.00	\$ 14.67
<b>Monthly Highs and Lows</b>		
October 2014	\$ 11.75	\$ 9.17
November 2014	\$ 15.70	\$ 9.69
December 2014	\$ 17.33	\$ 12.28
January 2015	\$ 23.80	\$ 14.67
February 2015	\$ 23.44	\$ 18.59
March 2015	\$ 28.00	\$ 21.06
April 2015 (through April 6)	\$ 35.50	\$ 22.51



On March 31, 2015 the closing sale price per share on The NASDAQ Global Select Market was \$24.32. On April 6, 2015, the closing sale price per share was \$33.61.

As of April 6, 2015, there was one shareholder of record in the United States, Cede & Co, the nominee for the Depository Trust Company, which held 63.8% of our outstanding ordinary shares.

**D. Selling Shareholders**

Not applicable.

**E. Dilution**

Not applicable.

**F. Expenses of the Issue**

**NOT APPLICABLE.**

**ITEM 10: ADDITIONAL INFORMATION**

**A. Share Capital**

Not applicable.

**B. Memorandum and Articles of Association**

We incorporate by reference into this Annual Report the description of our amended articles of association contained in our F-3 registration statement (File No. 333-202456) filed with the SEC on March 3, 2015.

**C. Material Contracts**

On July 15, 2014 we signed and on July 31, 2014 we closed an agreement to acquire all shares of InoCard GmbH. InoCard was founded in December 2013 as a spin-off of the University of Heidelberg, and is an early-stage biotechnology company focused on the development of gene therapy approaches for cardiac disease. InoCard has developed a novel gene therapy through preclinical proof of concept, for the one-time treatment of congestive heart failure (CHF). InoCard founders Prof. Patrick Most and Prof. Hugo Katus have joined uniQure as Managing Director of uniQure in Germany and Chairman of the Scientific Advisory Board, for Cardiovascular Diseases, respectively.

Under the terms of the agreement, the Company has paid the InoCard shareholders an upfront payment of approximately €3,000,000 (€1,500,000 in cash and €1,500,000 in uniQure shares (189,982 shares at closing of the transaction), and will receive a further €14,500,000 in success-based milestone payments upon achieving certain clinical and regulatory targets. Upon a successful commercial launch of a developed product, the sellers will further receive a royalty payment of 0.5% of the net product sales. The amount of the €14,500,000 in milestones is payable, at the Company's sole discretion, in either cash or a variable number of Company shares, based on the then current stock price

Other than the InoCard transaction, we have not entered into any material contracts other than as described under "Information on the Company—Business Overview—Intellectual Property", "—Collaborations—Bristol-Myers Squibb Collaboration", "—Collaborations—Early-Stage Collaborations: Chiesi Commercialization and Development Agreement," and "—Collaborations—Early-Stage Collaborations—4D Molecular Therapeutics", or in the ordinary course of business.

## D. Exchange Controls

Under existing laws of the Netherlands, there are no exchange controls applicable to the transfer to persons outside of the Netherlands of dividends or other distributions with respect to, or of the proceeds from the sale of, shares of a Dutch company.

## E. Taxation

### *Taxation in the Netherlands*

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 particular holder of our ordinary shares (a "Shareholder"). Shareholders are advised to consult their tax counsel with respect to the tax consequences of acquiring, holding and/or disposing of ordinary shares. 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:
  1. 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.
  2. 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.
  3. 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.
- A Shareholder who is a qualifying non-resident taxpayer within the meaning of article 7.8, paragraph 6, of the Dutch Income Tax Act 2001.

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 organizer ("Settlor"), 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 annual report, 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, deemed and constructive distributions, and repayments of paid-in capital which are not recognized for Dutch dividend withholding tax purposes;
- 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:

1. 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;
2. anyone or more of the following threshold conditions are satisfied:
  - a. 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
  - b. 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
  - c. 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
  - d. 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
3. 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 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, a Shareholder that is a corporation, another entity with a capital divided into shares, a cooperative (association), or another legal entity that has an enterprise to which the ordinary shares are attributable, that is 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 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 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 Shareholder, that is not an individual, and 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:

1. 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
2. such Shareholder 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
3. such Shareholder 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 (opschortende voorwaarde) 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 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 our ordinary shares. This summary is based on current provisions of the Code, existing, final, temporary and proposed U.S. Treasury Regulations, administrative rulings and judicial decisions, in each case as available on the date of this annual report. 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 provide assurance 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.** A "United States person," within the meaning of the Code, that is an individual, an estate or a nonexempt trust is generally subject to a 3.8% surtax on the lesser of (i) the United States person's "net investment income" for the year and (ii) the excess of the United States person's "modified adjusted gross income" for that year over a threshold (which, in the case of an individual, will be between \$125,000 and \$250,000, depending on the individual's U.S. tax filing status). A U.S. holder's net investment income generally will include, among other things, dividends on, and gains from the sale or other taxable disposition of, our ordinary shares, unless (with certain exceptions) those dividends or gains are derived in the ordinary course of a trade or business. Net investment income may be reduced by deductions properly allocable thereto; however, the U.S. foreign tax credit may not be available to reduce the surtax.

**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 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 2014 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. However, 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 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 our cash.

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. As long as our ordinary shares are regularly traded on the NASDAQ Global Select Market or another "qualified exchange," 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.

#### **F. Dividends and Paying Agents**

Not applicable.

#### **G. Statements by Experts**

Not applicable.

#### **H. Documents on Display**

We are subject to the periodic reporting and other informational requirements of the Securities Exchange Act of 1934, as amended, or the Exchange Act. Under the Exchange Act, we are required to file reports and other information with the SEC. Specifically, we are required to file annually a Form 20-F no later than four months after the close of each fiscal year, which is December 31. Copies of reports and other information, when so filed, may be inspected without charge and may be obtained

at prescribed rates at the public reference facilities maintained by the Securities and Exchange Commission at Judiciary Plaza, 100 F Street, N.E., Washington, D.C. 20549, and at the regional office of the Securities and Exchange Commission located at Citicorp Center, 500 West Madison Street, Suite 1400, Chicago, Illinois 60661. The public may obtain information regarding the Washington, D.C. Public Reference Room by calling the Commission at 1-800-SEC-0330. The SEC also maintains a web site at [www.sec.gov](http://www.sec.gov) that contains reports, proxy and information statements, and other information regarding registrants that make electronic filings with the SEC using its EDGAR system. As a foreign private issuer, we are exempt from the rules under the Exchange Act prescribing the furnishing and content of quarterly reports and proxy statements, and officers, directors and major shareholders are exempt from the reporting and short-swing profit recovery provisions contained in Section 16 of the Exchange Act.

**I. Subsidiary Information**

Not applicable.

**ITEM 11: QUANTITATIVE AND QUALITATIVE DISCLOSURES ABOUT MARKET RISK**

See "Operating and Financial Review and Prospects—Quantitative and Qualitative Disclosures about Market Risk."

**ITEM 12: DESCRIPTION OF SECURITIES OTHER THAN EQUITY SECURITIES**

Not applicable.

## PART II

### ITEM 13 DEFAULTS, DIVIDEND ARREARAGES AND DELINQUENCIES

Not applicable.

### ITEM 14: MATERIAL MODIFICATION TO THE RIGHTS OF SECURITY HOLDERS AND USE OF PROCEEDS

#### A. Material Modifications to the Rights of Securities Holders

Not applicable.

#### B. Use of Proceeds

The following "Use of Proceeds" information relates to our initial public offering, at \$17.00 per ordinary share, of 5,400,000 ordinary shares. The aggregate offering price was \$91,800,000, before underwriting discounts and commissions and offering expenses. The registration statement on Form F-1 (File No. 333-193158) for our initial public offering was declared effective by the SEC on February 4, 2013. Jefferies LLC, Leerink Partners LLC and Piper Jaffray & Co. were the underwriters for our initial public offering.

We received proceeds of \$85.4 million (€62.6 million) from our initial public offering, net of underwriting discounts and commissions but before expenses. No offering expenses were paid directly or indirectly to any of our directors or officers (or their associates) or persons owning 10.0% or more of any class of our equity securities or to any other affiliates.

As of December 31, 2014, we have used approximately \$50.2 (€38.0 million) of the net offering proceeds. Of this amount, approximately \$19.6 million (€14.9 million) was used primarily to fund the further build out of our Lexington, Massachusetts manufacturing facility, including a build-up in staff, and approximately \$30.6 million (€23.1 million) was used on the further development of AMT-060 (hemophilia B), research and development, building our product platform and advancing our pipeline of preclinical product candidates, and on working capital and general corporate purposes.

We are holding a significant portion of the balance of the net proceeds from the offering in interest-bearing money market accounts and prime money market funds. There has been no material change in our planned use of the balance of the net proceeds from the offering described in the Prospectus.

### ITEM 15: CONTROL AND PROCEDURES

#### *Disclosure Controls and Procedures*

Our management, with the participation of our chief executive officer and chief financial officer, evaluated the effectiveness of our disclosure controls and procedures as of December 31, 2014. The term "disclosure controls and procedures," as defined in Rules 13a-15(e) and 15d-15(e) under the Exchange Act, means controls and other procedures of a company that are designed to ensure that information required to be disclosed by a company in the reports that it files or submits under the Exchange Act is recorded, processed, summarized and reported, within the time periods specified in the SEC's rules and forms and that such information required to be disclosed by us in the reports that we file or submit under the Exchange Act is accumulated and communicated to our management, including our principal executive and principal financial officers, as appropriate to allow timely decisions regarding required disclosures. Based on the evaluation of our company's disclosure controls and procedures as of December 31, 2014, our chief executive officer and chief financial officer concluded that, as of such date, our company's disclosure controls and procedures were not effective as a result of the material weaknesses in internal control described below.

## ***Management's Annual Report on Internal Control over Financial Reporting***

Our management is responsible for establishing and maintaining adequate "internal control over financial reporting," as defined in Rules 13a-15(f) and 15d-15(f) under the Exchange Act. This rule defines internal control over financial reporting as a process designed by, or under the supervision of, a company's chief executive officer and chief financial officer and effected by our board of directors, management and other personnel, to provide reasonable assurance regarding the reliability of financial reporting and the preparation of financial statements for external purposes in accordance with generally accepted accounting principles and includes those policies and procedures that (1) pertain to the maintenance of records that, in reasonable detail, accurately and fairly reflect the transactions and dispositions of the assets of the company; (2) provide reasonable assurance that transactions are recorded as necessary to permit preparation of financial statements in accordance with generally accepted accounting principles, and that receipts and expenditures of the company are being made only in accordance with authorizations of management and directors of the company; and (3) provide reasonable assurance regarding prevention or timely detection of unauthorized acquisition, use, or disposition of the company's assets that could have a material effect on the financial statements.

Management assessed the effectiveness of our internal control over financial reporting as of December 31, 2014. This assessment was performed under the direction and supervision of our chief executive officer and chief financial officer, and based on criteria established in Internal Control—Integrated Framework (2013) issued by the Committee of Sponsoring Organizations of the Treadway Commission (COSO). Management has identified three control deficiencies that represent material weaknesses. A material weakness is a control deficiency, or a combination of control deficiencies in Internal Control over Financial Reporting, such that there is a reasonable possibility that a material misstatement of the annual or interim financial statements will not be prevented or detected. These control deficiencies could result in a misstatement of the financial statement accounts or related disclosures that would result in a material misstatement in the annual or interim consolidated financial statements that would not be presented or detected on a timely basis. Accordingly, management has determined that these control deficiencies constitute material weaknesses. Specifically, the following material weaknesses were identified:

- a lack of sufficient accounting resources required to fulfill IFRS and SEC reporting requirements;
- a lack of sufficient segregation of duties given the size of our finance and accounting team; and
- a lack of adequate closing procedures, supporting documentation and review.

Because of these material weaknesses, management concluded that we did not maintain effective internal control over financial reporting as of December 31, 2014 based on criteria described in internal control- Integrated framework(2013) issued by the COSO. We have implemented and are continuing to implement various measures to address the material weaknesses identified; these measures are outlined below.

### ***Remediation Plan***

We continue to evaluate our internal control over financial reporting and are taking several remedial actions to address the material weaknesses that have been identified. To this end, in particular, we hired our Chief Financial Officer on January 1, 2015 and have added additional staff within the finance department who have external reporting and IFRS experience, and experience with establishing appropriate financial reporting policies and procedures.

Moreover, we have engaged an external consultant to assist us to improve our corporate governance and internal control procedures and to help us design and implement a structured control environment for complying with the Sarbanes-Oxley Act of 2013.

We plan to implement formal independent reviewing controls over the access to our network and all systems critical for our financial reporting. During 2015 we plan to engage with IT specialists to implement adequate segregation of duties for all systems critical for financial reporting.

During 2015, our management intends to improve our closing checklist and document relevant standard operating procedures in order to enforce compliance and timeliness of our closing procedures. In addition management plans to strengthen and further formalize internal controls over manual journal entry controls, independent quarterly reviewing of balance sheet account reconciliations and budget/actuals comparisons.

We plan 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, nor can we ensure by what date they will be, fully remediated.

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 "Information on the Company—Risk Factors—Risks Related to 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."

#### ***Changes in internal control over financial reporting***

There has been no change in our internal control over financial reporting (as defined in Rule 13a-15(f) under the Exchange Act) during the period covered by this annual report that has materially affected, or is reasonably likely to materially affect, internal control over financial reporting.

#### **ITEM 16A: AUDIT COMMITTEE FINANCIAL EXPERT**

Mr. Ferdinand Verdonck and Mr. Will Lewis, independent directors and members of the Audit Committee, qualify as an "audit committee financial expert," as defined in Item 16A of Form 20-F and as determined by our supervisory board and management board.

#### **ITEM 16B: CODE OF ETHICS**

We have adopted a written code of ethics applicable to supervisory and managing directors, members of senior management and employees of the company and any of the company's direct and indirect subsidiaries. Our code of ethics is posted on our company website at:  
[http://www.unique.com/uploads/Exhibit%20I%20\\_%20uniQure%20Code%20of%20Business%20Conduct%20and%20Ethics\\_\(115245793\)\\_5\).pdf](http://www.unique.com/uploads/Exhibit%20I%20_%20uniQure%20Code%20of%20Business%20Conduct%20and%20Ethics_(115245793)_5).pdf)

Any amendments to our code of ethics will be disclosed on our website within five business days of the amendment.

**ITEM 16C: PRINCIPAL ACCOUNTANT FEES AND SERVICES**

The following table sets forth, for each of the years indicated, the fees billed by our independent public accountants and the percentage of each of the fees out of the total amount billed by the accountants.

	Year ended December 31, 2012		Year ended December 31, 2013		Year ended December 31, 2014	
	EUR'000	%	EUR'000	%	EUR'000	%
Audit Fees	65	93%	1,021	98%	635	96%
Audit-related Fees	—	0%	—	0%	—	0%
Tax Fees	5	7%	20	2%	29	4%
Total	70	100%	1,041	100%	664	100%

**Audit Fees** are defined as the standard audit work that needs to be performed each year in order to issue opinions on our consolidated financial statements and to issue reports on our local statutory financial statements. Also included are services that can only be provided by our auditor, such as reviews of quarterly financial results, consents and comfort letters and any other audit services required for SEC or other regulatory filings.

**Audit Related Fees** include those other assurance services provided by the independent auditor but not restricted to those that can only be provided by the auditor signing the audit report.

**Tax Fees** relate to the aggregated fees for services rendered on tax compliance.

**Pre-Approval Policies and Procedures for Non-Audit Services**

Our Audit Committee has adopted a policy pursuant to which we will not engage our auditors to perform any non-audit services unless the audit committee pre-approves the service, effective for the period following the completion of the IPO.

**ITEM 16D: EXEMPTIONS FROM THE LISTING REQUIREMENTS AND STANDARDS FOR AUDIT COMMITTEES**

Not applicable.

**ITEM 16E: PURCHASES OF EQUITY SECURITIES BY THE ISSUER AND AFFILIATED PURCHASERS**

None.

**ITEM 16F: CHANGE IN REGISTRANTS CERTIFYING ACCOUNTANT**

None.

**ITEM 16G: CORPORATE GOVERNANCE**

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 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 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.

#### **ITEM 16H: MINE SAFETY DISCLOSURE**

Not applicable.

### **PART III**

#### **ITEM 17: FINANCIAL STATEMENTS**

See "Financial Statements."

#### **ITEM 18: FINANCIAL STATEMENTS**

See the Financial Statements beginning on page F-1.

**ITEM 19: EXHIBITS**

<b>Exhibit No.</b>	<b>Description</b>
1.1	Amended Articles of Association of the Company (incorporated by reference to Exhibit 1.1 to the Company's 2014 Annual Report on Form 20-F (file no. 001-36294) filed with the Securities and Exchange Commission).
4.1†	Patent License Agreement (L-107-2007), effective as of May 2, 2007, by and between the Company and the National Institutes of Health, as amended on December 31, 2009, May 31, 2013 and November 11, 2013 (incorporated by reference to Exhibit 10.1 of the Company's registration statement on form F-1 (file no. 333-193158) filed with the Securities and Exchange Commission).
4.2†	Patent License Agreement (L-116-2011), effective as of August 10, 2011, by and between the Company and National Institutes of Health, as amended on May 31, 2013 and November 11, 2013 (incorporated by reference to Exhibit 10.2 of the Company's registration statement on form F-1 (file no. 333-193158) filed with the Securities and Exchange Commission).
4.3†	License Agreement, effective as of March 22, 2007, by and between the Company and Protein Sciences Corporation, as amended on June 13, 2012 (Incorporated by reference to Exhibit 10.3 of the Company's registration statement on form F-1 (file no. 333-193158) filed with the Securities and Exchange Commission.)
4.4†	Agreement, dated June 16, 2006, by and among the Company, Academish Medisch Centrum and Beheersmaatschappij Dienstverlening En Deelneming Azua (incorporated by reference to Exhibit 10.4 of the Company's registration statement on form F-1 (file no. 333-193158) filed with the Securities and Exchange Commission).
4.5†	Sublicense and Research Agreement, effective June 18, 2001, by and between the Company and Xenon Genetics Inc., as amended (incorporated by reference to Exhibit 10.5 of the Company's registration statement on form F-1 (file no. 333-193158) filed with the Securities and Exchange Commission.).
4.6†	License Agreement, effective as of December 20, 2006, between the Company and Aventis Pharma S.A., as amended on June 28, 2013 (incorporated by reference to Exhibit 10.6 of the Company's registration statement on form F-1 (file no. 333-193158) filed with the Securities and Exchange Commission).
4.7†	Non-Exclusive License Agreement, effective as of September 3, 2010, by and between the Company and Asklēpios Biopharmaceutical, Inc. (incorporated by reference to Exhibit 10.7 of the Company's registration statement on form F-1 (file no. 333-193158) filed with the Securities and Exchange Commission).
4.8†	License Agreement, dated February 8, 2008, by and between the Company and Salk Institute for Biological Studies (incorporated by reference to Exhibit 10.8 of the Company's registration statement on form F-1 (file no. 333-193158) filed with the Securities and Exchange Commission).
4.9†	License Agreement, dated December 5, 2006, by and between the Company and AmpliPhi Biosciences, Inc., as amended on June 28, 2013 (incorporated by reference to Exhibit 10.9 of the Company's registration statement on form F-1 (file no. 333-193158) filed with the Securities and Exchange Commission).

Exhibit No.	Description
4.10†	Exclusive License Agreement, effective as of July 7, 2008, by and between the Company and St. Jude Children's Research Hospital, Inc., as amended on July 12, 2012 (incorporated by reference to Exhibit 10.10 of the Company's registration statement on form F-1 (file no. 333-193158) filed with the Securities and Exchange Commission).
4.11†	Co-Development and License Agreement, entered into as of April 29, 2013, by and between the Company and Chiesi Farmaceutici S.p.A. (incorporated by reference to Exhibit 10.11 of the Company's registration statement on form F-1 (file no. 333-193158) filed with the Securities and Exchange Commission).
4.12†	Commercialization Agreement, entered into as of April 29, 2013, by and between the Company and Chiesi Farmaceutici S.p.A. (incorporated by reference to Exhibit 10.12 of the Company's registration statement on form F-1 (file no. 333-193158) filed with the Securities and Exchange Commission).
4.13†	License Agreement, dated as of May 21, 2010, by and among the Company, Fundacion para la Investigacion Medica Aplicada, Proyecto de Biomedicina CIMA S.L. and Digna Biotech, S.L. (incorporated by reference to Exhibit 10.13 of the Company's registration statement on form F-1 (file no. 333-193158) filed with the Securities and Exchange Commission).
4.14†	Development and Manufacturing Agreement, effective as of January 7, 2011, by and between the Company and Institut Pasteur, as amended on January 7, 2011 (incorporated by reference to Exhibit 10.14 of the Company's registration statement on form F-1 (file no. 333-193158) filed with the Securities and Exchange Commission).
4.15†	License Agreement, effective as of November 30, 2010, by and between the Company and Amgen Inc. (incorporated by reference to Exhibit 10.15 of the Company's registration statement on form F-1 (file no. 333-193158) filed with the Securities and Exchange Commission).
4.16†	Data License Agreement, effective June 12, 2012, by and between the Company and The Regents of the University of California, acting through its Office of Technology management, University of California, San Francisco (incorporated by reference to Exhibit 10.16 of the Company's registration statement on form F-1 (file no. 333-193158) filed with the Securities and Exchange Commission).
4.18	Warrant Agreement, dated as of September 20, 2013, by and among the Company, uniQure Biopharma B.V. and Hercules Technology Growth Capital, Inc. (incorporated by reference to Exhibit 10.18 of the Company's registration statement on form F-1 (file no. 333-193158) filed with the Securities and Exchange Commission).
4.19	Subscription Agreement, dated as of April 29, 2013, by and among Chiesi Farmaceutici S.p.A and the Company (incorporated by reference to Exhibit 10.19 of the Company's registration statement on form F-1 (file no. 333-193158) filed with the Securities and Exchange Commission).
4.20	Lease relating to Meibergdreef 45, 57 and 61, dated as of July 1, 2012, by and among Academisch Medisch Centrum and uniQure biopharma B.V. (incorporated by reference to Exhibit 10.26 of the Company's registration statement on form F-1 (file no. 333-193158) filed with the Securities and Exchange Commission).

Exhibit No.	Description
4.21	Lease relating to 113 Hartwell Avenue, Lexington, Massachusetts, dated as of July 24, 2013, by and between the Company and King113 Hartwell LLC (incorporated by reference to Exhibit 10.28 of the Company's registration statement on form F-1 (file no. 333-193158) filed with the Securities and Exchange Commission).
4.22	Business Acquisition Agreement, dated as of February 16, 2012, by and among Amsterdam Molecular Therapeutics (AMT) Holding N.V., the Company and the other Parties listed therein (incorporated by reference to Exhibit 10.29 of the Company's registration statement on form F-1 (file no. 333-193158) filed with the Securities and Exchange Commission).
4.23	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. (incorporated by reference to Exhibit 10.30 of the Company's registration statement on form F-1 (file no. 333-193158) filed with the Securities and Exchange Commission).
4.24	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. (incorporated by reference to Exhibit 10.31 of the Company's registration statement on form F-1 (file no. 333-193158) filed with the Securities and Exchange Commission).
4.25†	Collaboration and License Agreement, dated January 17, 2014, by and between uniQure biopharma B.V. and 4D Molecular Therapeutics, LLC (incorporated by reference to Exhibit 10.32 of the Company's registration statement on form F-1 (file no. 333-193158) filed with the Securities and Exchange Commission).
4.26	Option Agreement, dated January 17, 2014, by and between the Company and Dr. David Kirn (incorporated by reference to Exhibit 10.33 of the Company's registration statement on form F-1 (file no. 333-193158) filed with the Securities and Exchange Commission).
4.27	Option Agreement, dated January 17, 2014, by and between the Company and Dr. David Schaffer (incorporated by reference to Exhibit 10.34 of the Company's registration statement on form F-1 (file no. 333-193158) filed with the Securities and Exchange Commission).
4.28	Commitment Letter pursuant to Collaboration Agreement, dated January 17, 2014, by the Company and acknowledged and agreed by 4D Molecular Therapeutics, LLC, Dr. David Schaffer and Dr. David Kirn (incorporated by reference to Exhibit 10.35 of the Company's registration statement on form F-1 (file no. 333-193158) filed with the Securities and Exchange Commission).
4.29*	Amended and Restated Loan and Security Agreement, dated as of June 26, 2014 by and among the Company, uniQure IP B.V., the Company's subsidiaries listed therein, and Hercules Technology Growth Capital, Inc.
4.30†*	Collaboration and License Agreement by and between uniQure Biopharma B.V. and Bristol-Myers Squibb Company dated April 6, 2015
4.31†*	Share Subscription Agreement by and between uniQure N.V. and Bristol-Myers Squibb Company dated April 6, 2015

<u>Exhibit No.</u>	<u>Description</u>
4.32†*	Investor Agreement by and between uniQure Biopharma B.V. and Bristol-Myers Squibb Company dated April 6, 2015
4.33†*	Seventh Collaboration Warrant Agreement dated April 6, 2015 issued to Bristol-Myers Squibb Company
4.34†*	Tenth Collaboration Warrant Agreement dated April 6, 2015 issued to Bristol-Myers Squibb Company
8.1*	Subsidiaries of the Company
12.1*	Certification by Principal Executive Officer Pursuant to Section 302 of the Sarbanes-Oxley Act of 2002
12.2*	Certification by Principal Financial Officer Pursuant to Section 302 of the Sarbanes-Oxley Act of 2002
13.1*	Certification by Principal Executive Officer and Principal Financial Officer Pursuant to Section 906 of the Sarbanes-Oxley Act of 2002
15.1*	Consent of Independent Registered Public Accounting Firm
†	Confidential treatment requested as to certain portions, which portions have been omitted and filed separately with the Securities and Exchange Commission
*	Filed herewith

**Signatures**

The Registrant hereby certifies that it meets all of the requirements for filing on Form 20-F and that it has duly caused and authorized the undersigned to sign this annual report on its behalf.

uniQure N.V.

By: /s/ JÖRN ALDAG  
Jörn Aldag  
*Managing Director/Chief Executive Officer*

Date: April 7, 2015

By: /s/ MATTHEW KAPUSTA  
Matthew Kapusta  
*Chief Financial Officer*

Date: April 7, 2015

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**UNIQUE N.V.**  
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## **REPORT OF INDEPENDENT REGISTERED PUBLIC ACCOUNTING FIRM**

To the Management Board and shareholders of uniQure N.V.:

In our opinion, the accompanying consolidated balance sheets and the related consolidated statements of comprehensive loss, of changes in (deficit)/equity and of cash flows present fairly, in all material respects, the financial position of uniQure N.V. and its subsidiaries at December 31, 2014 and December 31, 2013, and the results of their operations and their cash flows for each of the three years in the period ended December 31, 2014 in conformity with International Financial Reporting Standards as issued by the International Accounting Standards Board. 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). 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.  
Utrecht, The Netherlands  
April 7, 2015

drs. A.C.M. van der Linden RA



**UNIQURE N.V.**  
**Consolidated Balance Sheets**  
**(€ in thousands)**

	<u>NOTE</u>	<u>DECEMBER 31, 2013</u>	<u>DECEMBER 31, 2014</u>
<b>Assets</b>			
<b>Non-current assets</b>			
Goodwill	5,9	—	1,342
Intangible assets other than Goodwill	5	7,775	16,368
Property, plant and equipment	6	2,614	19,667
Other non-current assets	7	923	1,022
<b>Total non-current assets</b>		<u>11,312</u>	<u>38,399</u>
<b>Current assets</b>			
Receivables from related parties	8	1,425	2,426
Trade and other receivables	8	1,557	1,542
Inventories	10	865	200
Cash and cash equivalents	11	23,810	53,219
<b>Total current assets</b>		<u>27,657</u>	<u>57,387</u>
<b>Total assets</b>		<u><b>38,969</b></u>	<u><b>95,786</b></u>
<b>Equity</b>			
Share capital		610	905
Share premium		142,459	206,111
Other reserves		6,536	17,149
Accumulated deficit		(144,041)	(181,081)
<b>Total equity</b>	<b>12</b>	<u><b>5,564</b></u>	<u><b>43,084</b></u>
<b>Liabilities</b>			
<b>Non-current liabilities</b>			
Borrowings	17	6,292	16,418
Financial lease liabilities	14	302	134
Deferred rent	29	680	5,658
Deferred revenue	18	15,679	15,387
Deferred tax liabilities	9,26	—	1,379
Contingent considerations	9	—	1,454
<b>Total non-current liabilities</b>		<u>22,953</u>	<u>40,430</u>
<b>Current liabilities</b>			
Trade and other payables	16	7,601	9,617
Debt to related party—derivative	15	722	645
Borrowings	17	633	—
Borrowings—derivative	17	217	207
Deferred rent	29	—	475
Deferred revenue	18	1,279	1,328
<b>Total current liabilities</b>		<u>10,452</u>	<u>12,272</u>
<b>Total liabilities</b>		<u><b>33,405</b></u>	<u><b>52,702</b></u>
<b>Total equity and liabilities</b>		<u><b>38,969</b></u>	<u><b>95,786</b></u>

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

UNIQUE N.V.

Consolidated Statements of Comprehensive Loss

(€ in thousands, except share and per share data)

	NOTE	YEARS ENDED DECEMBER 31,		
		2012	2013	2014
License revenues	18	—	440	883
Collaboration revenues	18	—	2,503	3,802
<b>Total revenues</b>		<b>—</b>	<b>2,943</b>	<b>4,685</b>
Cost of goods sold		—	(800)	—
Other income	19	649	585	773
Research and development expenses	21	(10,231)	(13,182)	(33,932)
Selling, general and administrative expenses	22	(4,564)	(11,628)	(11,167)
Other gains / (losses)—net	20	(45)	(453)	5,807
<b>Total operating costs</b>		<b>(14,840)</b>	<b>(25,263)</b>	<b>(39,292)</b>
<b>Operating result</b>		<b>(14,191)</b>	<b>(22,535)</b>	<b>(33,834)</b>
Finance income	25	22	102	254
Finance expense	25	(547)	(4,387)	(3,460)
<b>Finance income/(expense)—net</b>		<b>(525)</b>	<b>(4,285)</b>	<b>(3,206)</b>
<b>Result before corporate income tax</b>		<b>(14,716)</b>	<b>(26,820)</b>	<b>(37,040)</b>
Corporate income taxes		—	—	—
<b>Net loss</b>		<b>(14,716)</b>	<b>(26,820)</b>	<b>(37,040)</b>
Items that may be subsequently reclassified to profit or loss	23	—	12	1,149
<b>Other comprehensive income</b>	23	<b>—</b>	<b>12</b>	<b>1,149</b>
<b>Total comprehensive loss</b>		<b>(14,716)</b>	<b>(26,808)</b>	<b>(35,891)</b>
Loss per share attributable to the equity holders of the Company during the year:				
<b>Basic and diluted loss per share</b>	27	<b>(1.70)</b>	<b>(2.48)</b>	<b>(2.16)</b>

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

UNIQUE N.V.

Consolidated Statements of Changes in (Deficit)/Equity

(€ in thousands)

	Note	ATTRIBUTABLE TO EQUITY HOLDERS OF THE COMPANY				
		Total Share Capital	Share Premium	Other Reserves	Accumulated Deficit	Total Equity/Deficit
<b>Balance at January 1, 2012</b>		<b>237</b>	<b>99,947</b>	<b>2,728</b>	<b>(105,505)</b>	<b>(2,593)</b>
Result for the period					(14,716)	(14,716)
Capital contributions	12	246	14,848	—		15,094
Share-based payments relating to AMT share option scheme.	12	—	—	259	—	259
Adjustment to reserves on expiration of the AMT option scheme.		—	—	(2,987)	2,987	—
Share-based payment expenses relating to the uniQure share option scheme		—	—	1,508	—	1,508
<b>Balance at December 31, 2012</b>		<b>483</b>	<b>114,795</b>	<b>1,508</b>	<b>(117,234)</b>	<b>(448)</b>
Result for the period		—	—	—	(26,820)	(26,820)
Other comprehensive income		—	—	—	12	12
<b>Total comprehensive loss</b>		<b>—</b>	<b>—</b>	<b>—</b>	<b>(26,808)</b>	<b>(26,808)</b>
Capital contributions	12	127	27,664	—	—	27,791
Result on conversion of loan	15	—	—	3,005	—	3,005
Share based payment/expense	13	—	—	2,023	—	2,023
<b>Balance at December 31, 2013</b>		<b>610</b>	<b>142,459</b>	<b>6,536</b>	<b>(144,041)</b>	<b>5,564</b>
Result for the period		—	—	—	(37,040)	(37,040)
Other comprehensive income	23	—	—	1,149	—	1,149
<b>Total comprehensive loss</b>		<b>—</b>	<b>—</b>	<b>1,149</b>	<b>(37,040)</b>	<b>(35,891)</b>
Proceeds from shares issued	12	295	64,320	—	—	64,615
Share issuance costs			(668)	—	—	(668)
Share based payment/expense	13	—	—	9,464	—	9,464
<b>Balance at December 31, 2014</b>	<b>12</b>	<b>905</b>	<b>206,111</b>	<b>17,149</b>	<b>(181,081)</b>	<b>43,084</b>

The notes are an integral part of these consolidated financial statements

UNIQUE N.V.

Consolidated Statement of Cash Flows

(€ in thousands)

		YEARS ENDED DECEMBER 31,		
	NOTE	2012	2013	2014
<b>Cash flow from operating activities</b>				
Net loss		(14,716)	(26,820)	(37,040)
Adjustments for:				
Depreciation	6	548	535	1,539
Lease incentive	29	—	134	5,452
Derivative result	11	(22)	2,113	(87)
Derivative result arising on early conversion of loan	11	464	1,333	—
Exchange result		45	49	(4,692)
Other non-cash items	9	—	—	153
Share-based expenses	13	1,767	2,023	9,464
Changes in other non-current assets		—	(923)	—
Changes in trade and other receivables	8	243	(1,439)	(952)
Movement in inventories	10	—	(865)	664
Changes in trade and other payables	16	180	359	(989)
Changes in deferred revenue and provisions		—	16,958	(242)
Movement in other liabilities		161	2,052	1,068
Interest (income) / expense		61	1,244	1,461
<b>Cash used in operations</b>		<b>(11,269)</b>	<b>(3,247)</b>	<b>(24,201)</b>
Interest paid		(8)	(889)	(1,224)
<b>Net cash used in operating activities</b>		<b>(11,277)</b>	<b>(4,136)</b>	<b>(25,425)</b>
<b>Cash flow from investing activities</b>				
Purchases of property, plant and equipment	6	(392)	(1,336)	(15,769)
Purchases of intangible assets	5	(553)	(4,652)	(3,367)
Interest received		113	17	148
Acquisition of businesses	9	—	—	(1,463)
<b>Net cash used in investing activities</b>		<b>(832)</b>	<b>(5,971)</b>	<b>(20,451)</b>
<b>Cash flow from financing activities</b>				
Capital contribution from shareholders	12	9,774	14,294	—
Proceeds from shares issued	12	—	—	63,097
Share issuance cost	12	—	—	(668)
Convertible loans drawn down	15	1,498	11,999	—
Proceeds from borrowings	17	—	7,492	7,184
Redemption of financial lease	14	—	(143)	(156)
<b>Net cash generated from financing activities</b>		<b>11,272</b>	<b>33,642</b>	<b>69,457</b>
<b>Net (decrease)/increase in cash, cash equivalents and other bank overdrafts</b>		<b>(837)</b>	<b>23,535</b>	<b>23,581</b>
Currency effect cash and cash equivalents		—	12	5,828
Cash, cash equivalents and bank overdrafts at beginning of the period		1,100	263	23,810
<b>Cash, cash equivalents and bank overdrafts at end of the period</b>	<b>11</b>	<b>263</b>	<b>23,810</b>	<b>53,219</b>

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

## UNIQUE N.V.

### Notes to Consolidated Financial Statements

For the Years Ended December 31, 2012, 2013 and 2014

#### 1. General information

##### uniQure N.V.

uniQure N.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.

##### 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.

On April 5, 2012, uniQure raised €6.0 million through an issue to Forbion of 1,954,395 newly-issued class A ordinary shares at a price of €3.07 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 five euro cent ("class C ordinary shares") was created on July 22, 2013. While the A, B and class C ordinary shares all had the same economic rights, the principal difference was that class A ordinary shares and class C ordinary shares were held directly by shareholders, whereas the class B ordinary shares were held by a trust foundation (*stichting administratiekantoor* (the "STAK")) on behalf of the uniQure Depositary Receipt holders; the STAK Trustees attend uniQure shareholder meetings on behalf of the uniQure DR holders and will follow voting instructions from the uniQure Depositary Receipt holders in respect of any resolutions at shareholder meetings.

These consolidated financial statements of the Company are prepared on a going concern basis taking into account the announcement on April 6, 2015 of the agreements the Company entered into with Bristol-Myers Squibb, or BMS, with financial terms consisting of guaranteed, near-term payments to uniQure of at least \$97 million, including an upfront payment of \$50 million to be made at the closing of the transaction. The closing of the transaction is expected to occur in the second quarter of 2015, subject to Hart-Scott-Rodino clearance and customary closing conditions, which are considered by management to be reasonably certain of being satisfied. An additional \$15 million payment is to be

**UNIQUE N.V.**

## Notes to Consolidated Financial Statements (Continued)

For the Years Ended December 31, 2012, 2013 and 2014

**1. General information (Continued)**

received within three months of the closing for the selection of three additional collaboration targets, in addition to the Company's program for congestive heart failure. In addition, an initial equity investment in uniQure will be made for a number of shares that will equal 4.9% of the total number of shares outstanding following such issuance, at a purchase price of \$33.84 per share, or at least \$32 million in total. This investment is expected to be completed in the second quarter of 2015. BMS is also obligated to make an additional equity investment in uniQure for a number of shares that will equal 5.0% of the total number of shares outstanding following such issuance by December 31, 2015.

On February 10, 2014, the Company converted from a private company with limited liability (besloten vennootschap met beperkte aansprakelijkheid) incorporated under the laws of the Netherlands into a public company with limited liability (naamloze vennootschap), and changed its legal name from uniQure B.V. to uniQure N.V., and reclassified its class A, B and C ordinary shares as ordinary shares.

**Organizational structure of the uniQure Group**

uniQure N.V. is the ultimate parent of the following group of entities:

Company name

uniQure biopharma B.V.  
uniQure IP B.V.  
uniQure Manufacturing B.V.  
uniQure Assay Development B.V.  
uniQure Research B.V.  
uniQure non clinical B.V.  
uniQure QA B.V.  
uniQure Process Development B.V.  
uniQure clinical B.V.  
Stichting participatie AMT(1)  
uniQure Inc.  
uniQure GmbH(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 July 2014 the Company acquired InoCard GmbH, renamed as uniQure GmbH in August 2014.

**Other matters**

In January 2014, the Company entered into a collaboration and license agreement with 4D Molecular Therapeutics ("4D") for the discovery and optimization of next-generation AAV vectors. Under this agreement, the Company has 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

**UNIQUE N.V.****Notes to Consolidated Financial Statements (Continued)**

For the Years Ended December 31, 2012, 2013 and 2014

**1. General information (Continued)**

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 the Company will fund, at a cost of approximately \$3.0 million in aggregate over three years, to identify next generation AAV vectors. The Company is 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, the Company has granted options to purchase an aggregate of 609,744 ordinary shares in connection with this collaboration, and will recognize resulting share-based payment expense over three years. To the extent that the collaboration is successful, the Company may also incur additional third party costs in developing any product candidates and also in preparing, filing and prosecuting additional patent applications.

On January 20, 2014, the shareholders of the Company approved, and on January 21, 2014 the supervisory board of the Company confirmed, a 5-for-1 consolidation of shares, which had the effect of a reverse share split, that became effective on January 31, 2014. All share, per-share and related information presented in these consolidated financial statements and accompanying footnotes has been retroactively adjusted, where applicable, to reflect the impact of the reverse share split.

On February 5, 2014 the Company successfully completed its initial public offering, placing 5,400,000 shares at \$17 per share, raising total gross proceeds of \$91,800,000 (€67,300,000) and net proceeds of \$85,374,000 (€62,621,000) after commissions but before expenses. At the time of the initial public offering all existing shareholders agreed to a 180 day lock-up period which expired on August 4, 2014.

On July 15, 2014 the Company signed and on July 31, 2014 the Company closed an agreement to acquire all shares of InoCard GmbH. InoCard was founded in December 2013 as a spin-off of the University of Heidelberg, and is an early-stage biotechnology company focused on the development of gene therapy approaches for cardiac disease. InoCard has developed a novel gene therapy through preclinical proof of concept, for the one-time treatment of congestive heart failure (CHF). InoCard founders Prof. Patrick Most and Prof. Hugo Katus have joined uniQure as Managing Director of uniQure in Germany and Chairman of the Scientific Advisory Board, for Cardiovascular Diseases, respectively. For further disclosures please refer to Note 9.

The Company's business is not subject to seasonal influences.

The consolidated financial statements were authorized for issue by the supervisory board on March 26, 2015.

**2. Summary of Significant Accounting Policies****Introductory notes on the basis of preparation and presentation of the financial statements**

The 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.

**UNIQUIRE N.V.**

## Notes to Consolidated Financial Statements (Continued)

For the Years Ended December 31, 2012, 2013 and 2014

**2. Summary of Significant Accounting Policies (Continued)****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.

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 the Company's functional currency Euro, except where otherwise indicated.

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.

As described in Note 1 above, the combination of uniQure and the AMT Business, completed on April 5, 2012 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 twelve months ended December 31, 2012 (including the results of AMT prior to its acquisition by uniQure) and for each of the following reporting periods.

**2.1.1 Changes in accounting policy and disclosures****(a) New and amended standards adopted by the Company**

The following standards and amendments to standards became effective for annual periods on January 1, 2014 and have been adopted by the Company in the preparation of the consolidated financial statements:

IFRS 10	Amended / Consolidated Financial Statements
IFRS 12	Amended / Disclosures of Interest in Other Entities
IAS 27	Amended / Consolidated and Separate Financial Statements
IAS 32	Amended / Financial Instruments: Presentation
IAS 36	Amended / Impairment of Assets
IAS 39	Amended / Financial Instruments: Recognition and Measurement
Improvements to IFRSs— 2010 - 2012 Cycle	Amendments to IFRS 13—Short-term receivables and payables
Improvements to IFRSs— 2011 - 2013 Cycle	Amendments to IFRS 1—Meaning of 'effective IFRSs'

**Investment Entities (Amendments to IFRS 10, IFRS 12 and IAS 27)**

These amendments provide an exception to the consolidation requirement for entities that meet the definition of an investment entity under IFRS 10 *Consolidated Financial Statements* and must be



**UNIQUE N.V.**

## Notes to Consolidated Financial Statements (Continued)

For the Years Ended December 31, 2012, 2013 and 2014

**2. Summary of Significant Accounting Policies (Continued)**

applied retrospectively, subject to certain transition relief. The exception to consolidation requires investment entities to account for subsidiaries at fair value through profit or loss. These amendments have no impact on the Company since none of the entities in the Company qualifies to be an investment entity under IFRS 10.

**Offsetting Financial Assets and Financial Liabilities—Amendments to IAS 32**

This amendment clarifies that the right of set-off must not be contingent on a future event. It must also be legally enforceable for all counterparties in the normal course of business, as well as in the event of default, insolvency or bankruptcy. The amendment also considers settlement mechanisms. The amendment did not have a significant effect on the Company's financial statements.

**Amendments to IAS 36, 'Impairment of assets', on the recoverable amount disclosures for non-financial assets.**

This amendment removed certain disclosures of the recoverable amount of CGUs which had been included in IAS 36 by the issue of IFRS 13. This amendment has no impact on the Company.

**Novation of Derivatives and Continuation of Hedge Accounting—Amendments to IAS 39**

These amendments provide relief from discontinuing hedge accounting when novation of a derivative designated as a hedging instrument meets certain criteria and retrospective application is required. These amendments have no impact on the Company as the Company has not novated its derivatives during the current or prior periods.

**Annual Improvements 2010-2012 Cycle**

In the 2010-2012 annual improvements cycle, the IASB issued seven amendments to six standards, which included an amendment to IFRS 13 Fair Value Measurement. The amendment to IFRS 13 is effective immediately and clarifies in the Basis for Conclusions that short-term receivables and payables with no stated interest rates can be measured at invoice amounts when the effect of discounting is immaterial. This amendment to IFRS 13 has no impact on the Company.

**Annual Improvements 2011-2013 Cycle**

In the 2011-2013 annual improvements cycle, the IASB issued four amendments to four standards, which included an amendment to IFRS 1 First-time Adoption of International Financial Reporting Standards. The amendment to IFRS 1 is effective immediately and clarifies in the Basis for Conclusions that an entity may choose to apply either a current standard or a new standard that is not yet mandatory, but permits early application, provided that either standard is applied consistently throughout the periods presented in the entity's first IFRS financial statements. This amendment to IFRS 1 has no impact on the Company since the Company is an existing IFRS preparer.

The adoption of these new standards and amendments did not materially impact the Company's financial position or results of operations. Other standards, amendments and interpretations which are effective for the financial year beginning on January 1, 2014 are not material to the Company.

**UNIQUE N.V.**

## Notes to Consolidated Financial Statements (Continued)

For the Years Ended December 31, 2012, 2013 and 2014

**2. Summary of Significant Accounting Policies (Continued)****(b) New and amended standards not yet adopted by the Company**

A number of new standards and amendments to standards and interpretations are effective for annual periods beginning after January 1, 2014, and have not been applied in preparing these consolidated financial statements. None of these is expected to have a significant effect on the consolidated financial statements of the Company except the following set out below:

IFRS 9, 'Financial instruments', addresses the classification, measurement and recognition of financial assets and financial liabilities. The complete version of IFRS 9 was issued in July 2014. It replaces the guidance in IAS 39 that relates to the classification and measurement of financial instruments. IFRS 9 retains but simplifies the mixed measurement model and establishes three primary measurement categories for financial assets: amortized cost, fair value through Other Comprehensive Income and fair value through P&L. The basis of classification depends on the entity's business model and the contractual cash flow characteristics of the financial asset. Investments in equity instruments are required to be measured at fair value through profit or loss with the irrevocable option at inception to present changes in fair value in other comprehensive Income not recycling. There is now a new expected credit losses model that replaces the incurred loss impairment model used in IAS 39. For financial liabilities there were no changes to classification and measurement except for the recognition of changes in own credit risk in other comprehensive income, for liabilities designated at fair value through profit or loss. IFRS 9 relaxes the requirements for hedge effectiveness by replacing the bright line hedge effectiveness tests. It requires an economic relationship between the hedged item and hedging instrument and for the 'hedged ratio' to be the same as the one management actually uses for risk management purposes. Contemporaneous documentation is still required but is different to that currently prepared under IAS 39. The standard is effective for accounting periods beginning on or after January 1, 2018. Early adoption is permitted. The Company is yet to assess IFRS 9's full impact.

IFRS 15, 'Revenue from contracts with customers' deals with revenue recognition and establishes principles for reporting useful information to users of financial statements about the nature, amount, timing and uncertainty of revenue and cash flows arising from an entity's contracts with customers. Revenue is recognized when a customer obtains control of a good or service and thus has the ability to direct the use and obtain the benefits from the good or service. The standard replaces IAS 18 'Revenue' and IAS 11 'Construction contracts' and related interpretations. The standard is effective for annual periods beginning on or after January 1, 2017 and earlier application is permitted. The Company is assessing the impact of IFRS 15.

There are no other IFRSs or IFRIC interpretations that are not yet effective that would be expected to have a material impact on the Company.

**2.2 Consolidation**

The consolidated financial statements comprise the financial statements of the Company and its subsidiaries as at December 31, 2014. Subsidiaries are all entities over which the Company has control. The Company controls an entity when the Company is exposed to, or has rights to, variable returns from its involvement with the entity and has the ability to affect those returns through its power over the 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.

**UNIQUE N.V.**

## Notes to Consolidated Financial Statements (Continued)

For the Years Ended December 31, 2012, 2013 and 2014

**2. Summary of Significant Accounting Policies (Continued)**

If the business combination is achieved in stages, the acquisition date carrying value of the acquirer's previously held equity interest in the acquiree is re-measured to fair value at the acquisition date; any gains or losses arising from such re-measurement are recognized in profit or loss.

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.

**2.2.1 Business Combinations**

The Company applies the acquisition method to account for business combinations. The consideration transferred for the acquisition of a subsidiary is the fair values of the assets transferred, the liabilities incurred to the former owners of the acquiree and the equity interests issued by the Company. The consideration transferred includes the fair value of any asset or liability resulting from a contingent consideration arrangement. Identifiable assets acquired and liabilities and contingent liabilities assumed in a business combination are measured initially at their fair values at the acquisition date. The Company recognizes any non-controlling interest in the acquiree on an acquisition-by-acquisition basis, either at fair value or at the non-controlling interest's proportionate share of the recognized amounts of acquiree's identifiable net assets. Acquisition-related costs are expensed as incurred and included in administrative expenses

If the business combination is achieved in stages, the acquisition date carrying value of the acquirer's previously held equity interest in the acquiree is re-measured to fair value at the acquisition date; any gains or losses arising from such re-measurement are recognized in profit or loss.

Any contingent consideration to be transferred by the acquirer will be recognized at fair value at the acquisition date. Contingent consideration classified as an asset or liability that is a financial instrument and within the scope of IAS 39 Financial Instruments: Recognition and Measurement, is measured at fair value with changes in fair value recognized either in either profit or loss or as a change to other comprehensive Income. If the contingent consideration is not within the scope of IAS 39, it is measured in accordance with the appropriate IFRS. Contingent consideration that is classified as equity is not remeasured and subsequent settlement is accounted for within equity.

**2.3 Current versus non-current classification**

The Company presents assets and liabilities in the statement of financial position based on current/non-current classification. An asset is current when it is:

- expected to be realized or intended to be sold or consumed in normal operating cycle;
- held primarily for the purpose of trading;
- expected to be realized within twelve months after the reporting period; or
- cash or cash equivalent unless restricted from being exchanged or used to settle a liability for at least twelve months after the reporting period. All other assets are classified as non-current.

A liability is current when it is:

- expected to be settled in normal operating cycle;

**UNIQURE N.V.**

## Notes to Consolidated Financial Statements (Continued)

For the Years Ended December 31, 2012, 2013 and 2014

**2. Summary of Significant Accounting Policies (Continued)**

- held primarily for the purpose of trading;
- due to be settled within twelve months after the reporting period; or
- there is no unconditional right to defer the settlement of the liability for at least twelve months after the reporting period.

The Company classifies all other liabilities as non-current.

Deferred tax assets and liabilities are classified as non-current assets and liabilities.

**2.4 Fair value measurement**

The Company measures financial instruments such as derivatives, and non-financial assets at fair value at each balance sheet date. Fair value related disclosures for financial instruments and non-financial assets that are measured at fair value or where fair values are disclosed are summarized in Note 3.3.

The Company uses valuation techniques that are appropriate in the circumstances and for which sufficient data are available to measure fair value, maximising the use of relevant observable inputs and minimising the use of unobservable inputs.

**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 Euro. The consolidated financial statements are presented in Euro, 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.

**(c) Group Companies**

On consolidation, the assets and liabilities of foreign operations are translated into euro at the rate of exchange prevailing at the reporting date and their statements of profit or loss are translated at exchange rates prevailing at the dates of the transactions. The exchange differences arising on translation for consolidation are recognized in Other Comprehensive income. As the intercompany funding of the Company's Lexington operations is neither planned nor likely to be settled in the foreseeable future, the associated foreign exchange effect is presented as Other Comprehensive Income

**UNIQURE N.V.**

## Notes to Consolidated Financial Statements (Continued)

For the Years Ended December 31, 2012, 2013 and 2014

**2. Summary of Significant Accounting Policies (Continued)**

in the Other Reserves section of the Company's equity. On disposal of a foreign operation, the component of Other Comprehensive Income relating to that particular foreign operation is recognized in profit or loss.

Any goodwill arising on the acquisition of a foreign operation and any fair value adjustments to the carrying amounts of assets and liabilities arising on the acquisition are treated as assets and liabilities of the foreign operation and translated at the closing rate of exchange at the reporting date.

**2.6 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. The acquisition of InoCard GmbH has not changed the Company's assessment of having only one operating segment.

**2.7 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.

Further details are set out in Note 11 below.

**2.8 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.

**UNIQUE N.V.**

## Notes to Consolidated Financial Statements (Continued)

For the Years Ended December 31, 2012, 2013 and 2014

**2. Summary of Significant Accounting Policies (Continued)****(c) Goodwill**

Goodwill arises on the acquisition of subsidiaries and represents the excess of the consideration transferred, the amount of any non-controlling interest in the acquiree and the acquisition-date fair value of any previous equity interest in the acquire over the fair value of the identifiable net assets acquired. If the total of consideration transferred, non-controlling interest recognized and previously held interest measured at fair value is less than the fair value of the net assets of the subsidiary acquired, in the case of a bargain purchase, the difference is recognized directly in the income statement.

For the purpose of impairment testing, goodwill acquired in a business combination is allocated to each of the CGUs, or groups of CGUs, that is expected to benefit from the synergies of the combination. Each unit or group of units to which the goodwill is allocated represents the lowest level within the entity at which the goodwill is monitored for internal management purposes. Goodwill is monitored at the operating segment level.

Goodwill impairment reviews are undertaken annually or more frequently if events or changes in circumstances indicate a potential impairment. The carrying value of the CGU containing the goodwill is compared to the recoverable amount, which is the higher of value in use and the fair value less costs of disposal. Any impairment is recognized immediately as an expense and is not subsequently reversed.

**(d) In-process research & development**

In-process research and development ("IPR&D") represents the fair value assigned to incomplete research projects that the Company acquires through business combinations which, at the time of acquisition, have not reached technical feasibility. The amounts are capitalized and are accounted for as indefinite-lived intangible assets, subject to impairment testing until completion or abandonment of the projects. Upon successful completion of each project, uniQure will make a determination as to the then useful life of the intangible asset, generally determined by the period in which the substantial majority of the cash flows are expected to be generated, and begin amortization.

**2.9 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

**UNIQURE N.V.**

## Notes to Consolidated Financial Statements (Continued)

For the Years Ended December 31, 2012, 2013 and 2014

**2. Summary of Significant Accounting Policies (Continued)**

- 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.

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 2.4.26 below.

**2.10 Impairment of Non-Financial Assets**

Assets that are not subject to amortization (whether or not they are ready for use) are tested annually or more frequent if impairment indicators exist, by first assessing qualitative factors to determine whether it is more likely than not that the fair value of the intangible asset is less than its carrying amount. 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. 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). For the purpose of the impairment review the Company determined the entire uniQure group is considered one cash generating unit, as we currently use all material assets in the development of our gene therapies and our management regularly reviews all activities of our group as a single component.

The impairment review methodology applied is based on the fair value less cost of disposal concept. In this concept we compare the enterprise value (calculated by multiplying the outstanding shares as per the valuation date by the stock price of a ordinary share) plus the Company's debt and less the Company's cash, with the book value of the cash-generating unit. 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. Non-financial assets, other than goodwill, that have been previously impaired are reviewed for possible reversal of the impairment at each subsequent reporting date.

**2.11 Financial instruments—initial recognition and subsequent measurement**

A financial instrument is any contract that gives rise to a financial asset of one entity and a financial liability or equity instrument of another entity.

- a) Financial assets, initial recognition and measurement

All financial assets, such as receivables and deposits, are recognized initially at fair value plus, in the case of financial assets not recorded at fair value through profit or loss, transaction costs that are attributable to the acquisition of the financial asset.

The Company assesses, at each reporting date, whether there is objective evidence that a financial asset or a Company of financial assets is impaired. An impairment exists if one or more events that has occurred since the initial recognition of the asset (an incurred 'loss event'), has an impact on the

**UNIQUE N.V.**

Notes to Consolidated Financial Statements (Continued)

For the Years Ended December 31, 2012, 2013 and 2014

**2. Summary of Significant Accounting Policies (Continued)**

estimated future cash flows of the financial asset or the group of financial assets that can be reliably estimated.

b) Financial liabilities, initial recognition and measurement

Financial liabilities are classified, at initial recognition, as financial liabilities at fair value through profit or loss, loans and borrowings, payables, as appropriate. All financial liabilities are recognized initially at fair value and, in the case of loans and borrowings and payables, net of directly attributable transaction costs.

The Company's financial liabilities include trade and other payables, loans and borrowings including bank overdrafts, financial guarantee contracts and derivative financial instruments.

c) Subsequent measurement

The measurement of financial liabilities depends on their classification. Financial liabilities at fair value through profit or loss include financial liabilities held for trading and financial liabilities designated upon initial recognition as at fair value through profit or loss. Financial assets at fair value through profit or loss are subsequently carried at fair value. Loans and receivables are subsequently carried at amortized cost using the effective interest method.

d) Contingent consideration

As part of existing and future purchase agreements and following a Purchase Price Allocation, the Company could present amounts for contingent consideration. These amounts will be reviewed at any reporting cycle and any changes to the fair value of the contingent consideration will be recognized in the statement of profit or loss.

**2.12 Financial Assets and Liabilities**

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.

**Non-derivative financial instruments**

*Cash and cash equivalents*

Cash and cash equivalents include 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.



**UNIQUE N.V.**

## Notes to Consolidated Financial Statements (Continued)

For the Years Ended December 31, 2012, 2013 and 2014

**2. Summary of Significant Accounting Policies (Continued)***Trade Receivables*

Trade receivables are amounts due from customers for license fee payments or services performed in the ordinary course of business. If collection is expected in one year or less (or in the normal operating cycle of the business if longer), they are classified as current assets. Trade receivables are recognized initially at fair value and subsequently measured at amortized cost using the effective interest method, less provision for impairment, if any.

*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.

**UNIQURE N.V.**

## Notes to Consolidated Financial Statements (Continued)

For the Years Ended December 31, 2012, 2013 and 2014

**2. Summary of Significant Accounting Policies (Continued)****2.13 Impairment of Financial Assets**

The Company assesses at the end of each reporting period whether there is objective evidence that a financial asset or group of financial assets is impaired. A financial asset or a group of financial assets is impaired and impairment losses are incurred only if there is objective evidence of impairment as a result of one or more events that occurred after the initial recognition of the asset (a 'loss event') and that loss event (or events) has an impact on the estimated future cash flows of the financial asset or group of financial assets that can be reliably estimated.

Evidence of impairment may include indications that the debtors or a group of debtors is experiencing significant financial difficulty, default or delinquency in interest or principal payments, the probability that they will enter bankruptcy or other financial reorganization, and where observable data indicate that there is a measurable decrease in the estimated future cash flows, such as changes in arrears or economic conditions that correlate with defaults.

For loans and receivables category, the amount of the loss is measured as the difference between the asset's carrying amount and the present value of estimated future cash flows (excluding future credit losses that have not been incurred) discounted at the financial asset's original effective interest rate. The carrying amount of the asset is reduced and the amount of the loss is recognized in the consolidated income statement. If a loan or held-to-maturity investment has a variable interest rate, the discount rate for measuring any impairment loss is the current effective interest rate determined under the contract. As a practical expedient, the Company may measure impairment on the basis of an instrument's fair value using an observable market price.

If, in a subsequent period, the amount of the impairment loss decreases and the decrease can be related objectively to an event occurring after the impairment was recognized (such as an improvement in the debtor's credit rating), the reversal of the previously recognized impairment loss is recognized in the consolidated income statement.

**2.14 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. The legally enforceable right must not be contingent on future events and must be enforceable in the normal course of business and in the event of default, insolvency or bankruptcy of the company or the counterparty

**2.15 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.

**UNIQUE N.V.**

## Notes to Consolidated Financial Statements (Continued)

For the Years Ended December 31, 2012, 2013 and 2014

**2. Summary of Significant Accounting Policies (Continued)****2.16 Equity**

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.

Incremental costs directly attributable to the issue of new ordinary shares or options are shown in equity as a deduction, net of tax, from the proceeds.

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 would qualify 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, then 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

**UNIQURE N.V.**

## Notes to Consolidated Financial Statements (Continued)

For the Years Ended December 31, 2012, 2013 and 2014

**2. Summary of Significant Accounting Policies (Continued)**

income statement. If the conversion option qualifies as an equity instrument, it is recognized in equity on issue date and not re-measured.

**2.17 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.

**2.18 Deferred Corporate Income Taxes**

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 on temporary differences arising between the tax bases of assets and liabilities and their carrying amounts in the consolidated financial statements. However, deferred tax liabilities are not recognized if they arise from the initial recognition of goodwill; deferred income tax is not accounted for if it arises from initial recognition of an asset or liability in a transaction other than a business combination that at the time of the transaction affects neither accounting nor taxable profit or loss. Deferred income tax is determined using tax rates (and laws) that have been enacted or substantively 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.

**2.19 Employee Benefits****(a) Pension Obligations**

uniQure operates a defined contribution pension plan for all employees at its Amsterdam facility in the Netherlands, which is funded by the Company through payments to an insurance company.

**UNIQURE N.V.**

## Notes to Consolidated Financial Statements (Continued)

For the Years Ended December 31, 2012, 2013 and 2014

**2. Summary of Significant Accounting Policies (Continued)**

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.

uniQure operates a qualified 401(k) Plan for all employees at its Lexington facility in the USA. The uniQure, Inc. 401(k) Plan is an employee contribution plan only, and there are no employer contributions currently being made. The uniQure Inc. 401(k) Plan offers both a before tax and after tax (Roth) component, which are subject to IRS statutory limits for each calendar year.

**(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.

**2.20 Share-Based Compensation****uniQure share option plans**

The Company operates two share-based payment plans (2012 Plan and 2014 Plan), that both are equity settled share option plans under which options have been granted in 2012 and 2013 (2012 Plan) and in 2014 (2014 Plan). The 2014 Option Plan is described in the Company's 2014 Incentive Plan that enables various awards such as the granting of options and Restricted Stock Units (RSU's).

The fair value of the options in exchange for the services received is recognized as an expense, with a corresponding adjustment to a reserve in equity. The total amount to be expensed over the vesting period, if any, is determined by reference to the fair value of the options granted and based on the share price at grant and the vesting conditions. 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 period, under the 2012 Plan is 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. Under the 2014 Plan, in principle the first 25% vests after one year from the initial grant date and the remainder vests in equal quarterly installments, straight line over year two, three and four.

**UNIQUIRE N.V.**

## Notes to Consolidated Financial Statements (Continued)

For the Years Ended December 31, 2012, 2013 and 2014

**2. Summary of Significant Accounting Policies (Continued)**

Following the agreement with Management of 4D Molecular Therapeutics, the Company has granted options to purchase an aggregate of 609,744 ordinary shares in connection with this collaboration, and will recognize resulting share-based payment expense over three years (4D Option Plan). The total amount to be expensed over the vesting period, if any, is determined by reference to the fair value of the options granted.

**Restricted Stock Units (RSU's)**

Under the 2014 Incentive Plan the Company granted in October 2014 RSU's to the CEO. All of these RSU's will vest on February 6, 2016. The fair value of this grant on the date of grant will be recognized straight-line in expense over the period from initial grant through to the vesting date, with a corresponding adjustment to equity.

At each balance sheet date, the Company revises its estimates of the number of RSU's 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.

**2.21 Provisions**

Provisions are recognized when uniQure has a present legal or constructive obligation as a result of past events, it is probable that an outflow of resources will be required to settle the obligation, and the amount can be reliably estimated.

Provisions are measured at the present value of the expenditures expected to be required to settle the obligation using a pre-tax rate that reflects current market assessments of the time value of money and the risks specific to the obligation. The increase in the provision due to passage of time is recognized as interest expense.

**2.22 Revenues**

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 Company. The Company recognizes revenue when the amount of revenue can be reliably measured; when 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.

**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 the income statement as revenue over the period of the development, commercialization, collaboration or the manufacturing obligation.

**UNIQUIRE N.V.**

## Notes to Consolidated Financial Statements (Continued)

For the Years Ended December 31, 2012, 2013 and 2014

**2. Summary of Significant Accounting Policies (Continued)****(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.

**2.23 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. Government grants relating to costs are deferred and recognized in the income statement over the period necessary to match them with the costs that they are intended to compensate.

**2.24 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.

**UNIQUIRE N.V.**

## Notes to Consolidated Financial Statements (Continued)

For the Years Ended December 31, 2012, 2013 and 2014

**2. Summary of Significant Accounting Policies (Continued)****2.25 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.

**2.26 Leases****(a) 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.

**(b) 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.

**2.27 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.

**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.



**UNIQUE N.V.**

## Notes to Consolidated Financial Statements (Continued)

For the Years Ended December 31, 2012, 2013 and 2014

**3. Financial Risk Management (Continued)**

Financial risk management is carried out by the finance department. The finance department identifies and evaluates financial risks and hedges these risks if deemed appropriate. The Company has continued to strengthen the finance department which is responsible for financial risk management, through the appointment of additional senior personnel. As disclosed under post balance sheet events, a new CFO joined the Company on January 1, 2015. There have been no changes in the Company's financial risk management policies, since December 31, 2013.

**(a) Market Risk****(i) Foreign exchange risk**

The Company operates internationally and is exposed to foreign exchange risk arising from various currency exposures, primarily with respect to the US dollar and Euros and to a lesser extent to the British Pound. Foreign exchange risk arises as the Company acquires certain materials and pays for certain licenses and other services in these currencies.

At December 31, 2014 there was a net amount of trade payables denominated in U.S. Dollars of €1.8 million. This is broken out in an amount in the books of the Dutch entity of €0.1 million (2013: €0.3 million) and an amount of €1.7 million (2013: €0.7 million) in the books of the US entity. At December 31, 2014 there was a net trade payable denominated in British Pounds of €0.3 million (2013: €0.1 million).

Foreign currency denominated trade receivables and trade payables are short term in nature (generally 30 to 45 days). As a result foreign exchange rate movements on trade receivables and trade payables, during the years presented had a sizable effect on the financial statements. The Company has certain investments in foreign operations, whose net assets are exposed to foreign currency translation risk. As of December 31, 2014 there was a significant effect on the Company's loss due to weakening of the functional currency against any foreign currency.

At December 31, 2014, if the euro had weakened 10 percent against the US dollar with all other variables held constant, post-tax profit for the year would have been €3.4 million higher (2013: €0.2 million), and other comprehensive income would have been €1.1 million higher (2013: €0.2 million). Conversely, if the euro had strengthened 10 percent against the US dollar with all other variables held constant, post-tax profit would have been €3.4 million lower (2013: €0.2 million), and other comprehensive income would have been €1.1 million lower (2013: €0.2 million).

The sensitivity in the 2014 net result to fluctuations in foreign currency exchange rates, is attributable to the fact that the majority of cash and cash equivalents at December 31, 2014, were held in US dollars. This is partly offset against the Hercules venture debt loan with a nominal value in US dollars of 20 million. The sensitivity in Other Comprehensive Income to fluctuations in exchange rates is related to the funding by the Dutch holding company of the investing and operating activities of the Company's U.S. based entity.

The Company is in the process of setting up a policy to manage the foreign exchange risk against the functional currency.

**UNIQURE N.V.**

## Notes to Consolidated Financial Statements (Continued)

For the Years Ended December 31, 2012, 2013 and 2014

**3. Financial Risk Management (Continued)****(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. In July 2013 the Company entered into an agreement with Hercules Technology Growth Capital for a \$10 million denominated loan, which was subsequently amended in July 2014 to increase to a total loan amount of \$20 million.

At December 31, 2014 if interest rates on borrowings had been 1.0% higher/lower with all other variables held constant, post-tax results for the year would have been €114,000 (2013: €42,000) lower/higher as a result of changes in the fair value of the borrowings. The effect of a change in interest rates of 1.0% on borrowings would have had an insignificant effect on post-tax results for the year as a result of changes in the fair value of the venture debt facility.

During 2013 uniQure had long-term interest bearing liabilities under the 2012 Convertible loan which was subsequently converted into 1,336,331 Class A ordinary shares on July 26, 2013. uniQure does not enter into any interest rate swaps.

**(b) Credit Risk**

Credit risk is managed on Company basis. Credit risk arises from cash and cash equivalents, derivative financial instruments and deposits with banks and financial institutions, as well as credit exposures to wholesale customers, including outstanding receivables and committed transactions.

The Company has currently no wholesale debtors other than Chiesi. Please refer to Note 18 and 30 for further information on the Company's relationship with Chiesi.

The security deposit under other non-current assets represents the amount the Company paid to the landlord in September 2013 in relation to the facility in Lexington, Massachusetts. The deposit is neither impaired nor past due.

# UNIQURE N.V.

Notes to Consolidated Financial Statements (Continued)

For the Years Ended December 31, 2012, 2013 and 2014

## 3. Financial Risk Management (Continued)

As of December 31, 2014 and December 31, 2013, the majority of uniQure's cash and cash equivalents were placed at the following banks:

(€ in thousands)	AS OF DECEMBER 31,			
	2013		2014	
	AMOUNT	CREDIT RATING	AMOUNT	CREDIT RATING
<b>Bank</b>				
Rabobank(1)	23,810	Aa2	53,117	Aa2
CommerzBank(1)(2)	—	—	102	Baa1
Total	<u>23,810</u>		<u>53,219</u>	

(1) Ratings are by Moody's

(2) In July 2014, the Company acquired InoCard, which holds an account with Commerzbank.

The policy to accept banks and financial institutions with a minimum rating of "A" has been adapted to also accept Commerzbank (with a Baa1 rating). InoCard, acquired by the Company in July 2014, holds an account with Commerzbank. There are no financial assets past due date or impaired. No credit limits were exceeded during the reporting period.

### (c) Liquidity Risk

Management considers uniQure's cash and cash equivalents as of December 31, 2014, when taken together with additional funds raised since that date following the collaboration with Bristol-Myers Squibb, are sufficient to carry out the business plans going forward 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 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

# UNIQUE N.V.

## Notes to Consolidated Financial Statements (Continued)

For the Years Ended December 31, 2012, 2013 and 2014

### 3. Financial Risk Management (Continued)

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	UNDEFINED
(€ in thousands)					
<b>At December 31, 2013</b>					
Borrowings (excl. Finance lease liabilities)	1,498	3,372	4,192	—	—
Financial lease liabilities	156	168	134	—	—
Trade and other payables	7,445	—	—	—	—
Derivatives	939	—	—	—	—
<b>Total</b>	<b>10,038</b>	<b>3,540</b>	<b>4,326</b>	<b>—</b>	<b>—</b>
<b>At December 31, 2014</b>					
Borrowings (excl. Finance lease liabilities)	1,710	7,773	11,480	—	—
Financial lease liabilities	168	134	—	—	—
Trade and other payables	9,449	—	—	—	—
Contingent consideration	—	—	—	—	14,500
Derivatives	852	—	—	—	—
<b>Total</b>	<b>12,179</b>	<b>7,907</b>	<b>11,480</b>	<b>—</b>	<b>14,500</b>

Due to uncertainty of timing of achieving milestones, the amount for contingent consideration is classified as undefined in time. When due, the amount can be settled either in cash or in a variable number of Company shares.

### 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.

### 3.3 Fair value estimation

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);

# UNIQUE N.V.

## Notes to Consolidated Financial Statements (Continued)

For the Years Ended December 31, 2012, 2013 and 2014

### 3. Financial Risk Management (Continued)

- 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).

As of December 31, 2014 and 2013 financial instruments at fair value through profit and loss amounted to a loss of €87,000 and €3,446,000 respectively, and comprised in 2013 of movements on the fair value of the derivative elements of convertible loans.

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.

Following the Initial Public Offering in February 2014, the measurement for the warrants is now a level 2 valuation and no longer a level 3 valuation, as our shares are currently traded on NASDAQ under the symbol "QURE" and the valuation of the warrants is derived from the quoted share price. The transfer from level 3 in the table below is presented and accounted for at the beginning of the accounting period.

	<u>LEVEL 1</u>	<u>LEVEL 2</u>	<u>LEVEL 3</u>	<u>TOTAL</u>
	(€ in thousands)			
<b>At December 31, 2013</b>				
Debt to related party—derivative (warrants)	—	—	722	722
Borrowings—derivative (warrants)	—	—	217	217
	<u>—</u>	<u>—</u>	<u>939</u>	<u>939</u>

	<u>LEVEL 1</u>	<u>LEVEL 2</u>	<u>LEVEL 3</u>	<u>TOTAL</u>
	(€ in thousands)			
<b>At December 31, 2014</b>				
Debt to related party—derivative (warrants)	—	645	—	645
Borrowings—derivative (warrants)	—	207	—	207
Contingent consideration	—	—	1,454	1,454
	<u>—</u>	<u>852</u>	<u>1,454</u>	<u>2,306</u>

# UNIQUE N.V.

## Notes to Consolidated Financial Statements (Continued)

For the Years Ended December 31, 2012, 2013 and 2014

### 3. Financial Risk Management (Continued)

	LEVEL 3		
	derivatives at fair value through profit or loss	Contingent consideration	Total Level 3
<b>Opening balance January 1, 2013</b>	132	—	132
Transfers to (from) level 3	366	—	366
Movement in equity on early conversion of loan	(3,005)	—	(3,005)
Losses recognized in profit or loss	3,446	—	3,446
<b>Closing balance at December 31, 2013</b>	<b>939</b>	<b>—</b>	<b>939</b>

	LEVEL 3		
	derivatives at fair value through profit or loss	Contingent consideration	Total Level 3
<b>Opening balance January 1, 2014</b>	939	—	939
Transfers to (from) level 3	(939)	—	(939)
Acquisition of InoCard GmbH (note 9)	—	1,301	1,301
Losses recognized in profit or loss	—	153	153
<b>Closing balance at December 31, 2014</b>	<b>—</b>	<b>1,454</b>	<b>1,454</b>

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.

#### Group valuation processes

The Fair Value of the Level 3 contingent consideration is estimated using a Discounted Cash Flow methodology, as the expected (i.e. probability-weighted) present value of the milestone payments and based on a discount rate of 30%. The fair value could change as the probability of the milestone payments changes, or due to the time value of money. 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.

### 4. Critical Accounting Estimates and Judgments

Estimates and judgments are continually evaluated and are based on historical experience and other factors, including expectations of future events that are believed to be reasonable under the circumstances.

#### 4.1 Critical accounting estimates and assumptions

The Company makes estimates and assumptions concerning the future. The resulting accounting estimates will, by definition, seldom equal the related actual results. The estimates and assumptions that have a significant risk of causing a material adjustment to the carrying amounts of assets and liabilities within the next financial year are addressed below.

**UNIQURE N.V.**

## Notes to Consolidated Financial Statements (Continued)

For the Years Ended December 31, 2012, 2013 and 2014

**4. Critical Accounting Estimates and Judgments (Continued)****Revenue recognition**

The Company has not generated any revenues from royalties or product sales through December 31, 2014.

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.

**Valuation of Warrants**

With the venture debt loan facility and after the conversion of the convertible loan in 2013 the Company is accounting for the valuation of warrants (total warrants as per December 31, 2014: 170,802 (2013: 170,802), with a corresponding carrying value of €852,000 (2013: €939,000). The fair value of the warrants is based on the Black-Scholes model. Assumptions are made on inputs such as time to maturity, the share price, volatility and risk free rate, in order to determine the fair value per warrant. In addition there is an assumption on foreign exchange to calculate the euro value of the Hercules warrants.

The effect, when some of these underlying parameters would deviate by 10% up or down is presented in the below table.

	Share Price	Volatility	Time to Maturity
-10%	701,000	791,000	821,000
<b>Base Case</b>	<b>852,000</b>	<b>852,000</b>	<b>852,000</b>
+10%	1,011,000	913,000	882,000

**UNIQURE N.V.**

## Notes to Consolidated Financial Statements (Continued)

For the Years Ended December 31, 2012, 2013 and 2014

**4. Critical Accounting Estimates and Judgments (Continued)****Share-based payments**

The Company as per the reporting date operates two equity settled share option plans. At the balance sheet date of December 31, 2014 a total of 2,596,532 options were granted and outstanding (2013: 1,691,844, 2012: 1,606,347) under these two plans. In addition the Company operates a plan for the management of 4D Molecular Therapeutics. At the balance sheet date of December 31, 2014, a total of 457,308 options were granted and outstanding. These plans are accounted for in accordance with the policy as stated in Note 2.4.18. The option pricing model used and the inputs to that model are described in Note 13 below.

In August 2014 the Company also granted Restricted Stock Units (RSU's). At the balance sheet date of December 31, 2014 a total of 179,068 RSU's were granted and outstanding (2013: nil).

For the periods ended December 31, 2012, 2013 and 2014 the recorded expenses for share based expenses were €1,767,000, €2,023,000 and €9,464,000 respectively. At the date of the IPO a total of 1,507,443 options vested in full.

**Corporate taxes**

The Company is subject to corporate taxes in the jurisdictions it is operating in. 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.

**In Process Research and Development (IPR&D)**

Following the InoCard transaction the Company recorded an IPR&D valued at acquisition date of €4,655,000 (2013: nil). As per the balance sheet date the Company tested for impairment and re-assessed the Fair Value for the IPR&D. Based on this test it was assessed that no impairment charge needed to be recorded.

**Goodwill**

In 2014, following the InoCard transaction the Company recorded a Goodwill amount of €1,342,000 (2013: nil). The Goodwill was derived from the Purchase Price Allocation where the IPR&D was reconciled to the deferred tax liability and the total consideration. The Impairment review performed at balance sheet date indicated no change to the amount as presented.

**Contingent Consideration**

In 2014, following the InoCard transaction the Company recorded a contingent consideration at acquisition date of €1,301,000 (2013: nil). A subsequent valuation of the fair value of the contingent consideration at balance sheet date the Company resulted in a contingent consideration of €1,454,000,



## UNIQUE N.V.

### Notes to Consolidated Financial Statements (Continued)

For the Years Ended December 31, 2012, 2013 and 2014

#### 4. Critical Accounting Estimates and Judgments (Continued)

by applying a discounted cash flow calculation, considered only the passing of time since the initial valuation.

Performing a sensitivity analysis on the fair value estimation on the contingent consideration whereby varying, next to the passing of time, the unobservable inputs such as the timing and Probability of Success (PoS) in achieving the milestones, gave the following overview as per December 31, 2014.

	<b>FV Estimation</b>	
<b>Initial valuation at acquisition date</b>	<b>1,301</b>	
Passing of time between initial valuation and reporting date, P&L impact	153	1,454
<b>Sensitivities applied, over and above passing of time:</b>	<b>Delta</b>	<b>FV</b>
Moving out of all milestones by 6 months	(8)	1,293
Increasing the POS for the first milestone by 20%	735	2,036
Decreasing the POS for the first milestone by 20%	(429)	872
Reducing the discount rate from 30% to 20%	734	2,035
Increasing the discount rate from 30% to 40%	(103)	1,198

In addition, the fair value of the contingent consideration is also affected by the timing of the commencement of products sales that will trigger further royalty payments to the former shareholders of InoCard. The POS sensitivity in the above table has an effect on the total POS used in the fair value calculation.

#### 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 position 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 European Union for

**UNIQUE N.V.**

## Notes to Consolidated Financial Statements (Continued)

For the Years Ended December 31, 2012, 2013 and 2014

**4. Critical Accounting Estimates and Judgments (Continued)**

Glybera, such authorization is subject to further conditions before first sales may be made in the European Union.

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. 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, the intangible asset will be carried at its cost less any accumulated amortization and any accumulated impairment losses (cost model). The remaining useful life over which the intangible will be amortized is estimated at approximately 18 years.

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

**UNIQURE N.V.**

## Notes to Consolidated Financial Statements (Continued)

For the Years Ended December 31, 2012, 2013 and 2014

**4. Critical Accounting Estimates and Judgments (Continued)**

December 31, 2014, management reviewed the carrying amount of these assets and determined that no adjustments to carrying values were required.

On assets that are not subject to amortization, the Company annually performs an impairment review based on the fair value less cost of disposal method. 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 European Union. The Company's future revenues from product revenue 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. 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.

Next to Glybera-related intangible assets, in the impairment review the Company also considered the intangible assets associated with other pipeline products and the manufacturing platform. New to the review in 2014 were the In Process Research and Development and Goodwill as they arose from the InoCard acquisition.

The Company has determined that no impairment charge is required for the year ended December 31, 2014. Performing a further sensitivity analysis on the fair value calculation (by for example, reducing the fair value per ordinary share by 20%, as used in the calculation of the enterprise value), did not change management's conclusion that no impairment charge was required.

**(d) 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 2014, 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.

# UNIQUE N.V.

Notes to Consolidated Financial Statements (Continued)

For the Years Ended December 31, 2012, 2013 and 2014

## 5. Intangible Assets

	LICENSE FEES	CAPITALIZATION OF DEVELOPMENT EXPENSES	IN-PROCESS RESEARCH & DEVELOPMENT (€ in thousands)	GOODWILL	TOTAL INTANGIBLE ASSETS
<b>As of January 1, 2013</b>					
Cost	3,278	—	—	—	3,278
Accumulated amortization and impairment	—	—	—	—	—
<b>Opening net book amount</b>	<b>3,278</b>	<b>—</b>	<b>—</b>	<b>—</b>	<b>3,278</b>
Additions	1,544	3,108	—	—	4,652
Reductions	(155)	—	—	—	(155)
Amortization charge	—	—	—	—	—
<b>Closing net book amount</b>	<b>4,667</b>	<b>3,108</b>	<b>—</b>	<b>—</b>	<b>7,775</b>
<b>At December 31, 2013</b>					
Cost	4,667	3,108	—	—	7,775
Accumulated amortization and impairment	—	—	—	—	—
<b>Net book amount</b>	<b>4,667</b>	<b>3,108</b>	<b>—</b>	<b>—</b>	<b>7,775</b>
<b>As of January 1, 2014</b>					
<b>Opening net book amount</b>	<b>4,667</b>	<b>3,108</b>	<b>—</b>	<b>—</b>	<b>7,775</b>
Additions	225	3,703	4,665	1,342	9,935
Reductions	—	—	—	—	—
Amortization charge	—	—	—	—	—
<b>Closing net book amount</b>	<b>4,892</b>	<b>6,811</b>	<b>4,665</b>	<b>1,342</b>	<b>17,710</b>
<b>At December 31, 2014</b>					
Cost	4,892	6,811	4,665	1,342	17,710
Accumulated amortization and impairment	—	—	—	—	—
<b>Net book amount</b>	<b>4,892</b>	<b>6,811</b>	<b>4,665</b>	<b>1,342</b>	<b>17,710</b>

In the years presented in these financial statements, no amortization expense was recorded because the related products for which licenses have been granted have, in case of Glybera, not seen their first commercial sale, or in relation to products under development, have not yet been approved for commercial sale by regulatory authorities. For the amount associated with Glybera amortization will start the month the first commercial sales of the approved product will be recorded.

## UNIQUE N.V.

### Notes to Consolidated Financial Statements (Continued)

For the Years Ended December 31, 2012, 2013 and 2014

#### 5. Intangible Assets (Continued)

##### Licenses

The net book amount of uniQure's licenses by licensor is set out below:

	DECEMBER 31,		
	2012	2013	2014
	(€ in thousands)		
Xenon	365	765	765
AmpliPhi	2,352	2,197	2,197
NIH	317	1,130	1,209
UCSF	244	244	244
St. Jude	—	250	250
Salk Institute	—	4	4
Protein Sciences Corporation	—	77	77
4D Molecular Therapeutics	—	—	146
<b>Total</b>	<b>3,278</b>	<b>4,667</b>	<b>4,892</b>

The amounts set out above arose as follows:

In June 2001, the Company obtained a sub-license from Xenon Genetics, Inc. ("Xenon"), which was approved by Xenon's licensor, The University of British Columbia. The sub-license was initially capitalized in the amount of €140,000. Xenon granted the Company 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 Company acquired a sub-license from Targeted Genetics Corporation (now renamed AmpliPhi Biosciences, Inc. ("AmpliPhi")). The sub-license was approved by AmpliPhi's licensor, The University of Pennsylvania. It is related to "AAV1 Vector" technology, and the recognized acquisition amount is €1,330,000, which was capitalized.

In 2007, the Company 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 Company capitalized licensing fees totaling €600,000 related to a license from the La Sapienza University of Rome ("La Sapienza") for technology for treatment for Duchenne Muscular Dystrophy and a license from the San Raffaele University of Milano for technology to be used in the treatment of Factor IX Hemophilia.

**UNIQUE N.V.**

## Notes to Consolidated Financial Statements (Continued)

For the Years Ended December 31, 2012, 2013 and 2014

**5. Intangible Assets (Continued)**

In 2009, the Company 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 Company terminated its research and license agreement with San Raffaele University of Milano. This expense had been capitalized as an intangible asset, and accordingly this amount has been written off (€300,000).

In 2011, the Company made and capitalized a payment to the NIH in the amount of €109,000 for a license to use AAV5.

During 2011, the Company stopped further development of its Duchenne Muscular Dystrophy program. At that time, the program had not met its scientific goals. Accordingly, the amount capitalized (€300,000) as an intangible asset in respect of the license from La Sapienza described above has been written off.

In 2012, the Company 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 Company also made and capitalized a payment to Xenon Pharmaceuticals Inc. of CAN\$ 200,000 (€155,000) in respect of Glybera's approval by EMA.

In 2012, the Company 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 June 2013, when the agreements with Chiesi became unconditional, the Company booked amounts related to amendment fees in relation to licenses granted to subcontractors for a total amount of €1,544,000, broken out as follows: Xenon €400,000, NIH €813,000, St. Jude € 250,000, Salk Institute €4,000 and Protein Sciences Corporation € 77,000. For the last three parties mentioned the Company incurred annual maintenance fees only in prior years.

On July 1, 2013, the Company altered the terms of the previous Glybera-related license agreement, entered into in 2012, with AmpliPhiBiosciences Corporation, reducing the capitalized amount by €155,000 (CAN\$200,000).

In January 2014, the Company made and capitalized a payment of \$200,000 (€146,000) in accordance with its financial obligations relating to the further development of vector technologies.

In October 2014, the Company made and capitalized a payment to the NIH in the amount of €79,000 for an amendment to the license to use adeno-associated viruses.

**Capitalization of Development Expenses**

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, the intangible asset will be carried at its cost less any accumulated amortization and any accumulated impairment losses (cost model). The estimated useful

**UNIQURE N.V.**

## Notes to Consolidated Financial Statements (Continued)

For the Years Ended December 31, 2012, 2013 and 2014

**5. Intangible Assets (Continued)**

life over which the intangible will be amortized is estimated approximately to be another 18 years ending in 2032; the date of expiration of the last intellectual property protection related to the manufacturing process. As at the December 31, 2014 balance sheet date the Company recorded a total of €6,811,000 (2013: €3,108,000) related to capitalized development costs for Glybera.

**In Process Research & Development (IPR&D)**

The presented IPR&D relates to the InoCard acquisition. UniQure GMBH (InoCard) is effectively a single-product business, fully focusing on the further development of gene therapy approaches for cardiac disease. As of the acquisition date the Company performed a purchase price allocation under IFRS 3 that resulted in an initial fair value assessment of the acquired IPR&D asset in a value of €4,665,000.

**Goodwill**

The InoCard acquisition and its Purchase Price Allocation calculation performed at initial recognition resulted in goodwill of €1,342,000. As the value of the company is considered to be fully represented by the fair value of the underlying asset in the sense that all cash flows generated by the company are attributable to the underlying asset, the economic goodwill is immaterial. However as the underlying asset, the IPR&D, due to its undeductability for tax purposes, generates a deferred tax liability, the Company has to account for goodwill. For further disclosures please refer to Note 9.

# UNIQUE N.V.

## Notes to Consolidated Financial Statements (Continued)

For the Years Ended December 31, 2012, 2013 and 2014

### 6. Property, Plant and Equipment

	LEASEHOLD IMPROVEMENTS	CONSTRUCTION IN PROCESS	LAB EQUIPMENT	OFFICE EQUIPMENT	TOTAL
	(€ in thousands)				
<b>As of January 1, 2013</b>					
Cost	1,264	—	2,959	879	5,102
Accumulated depreciation	(666)	—	(2,689)	(562)	–3,917
<b>Opening net book amount</b>	<b>598</b>	<b>—</b>	<b>270</b>	<b>317</b>	<b>1,185</b>
Additions	—	1,285	175	504	1,964
Depreciation charge	(185)	—	(124)	(226)	(535)
<b>Closing net book amount</b>	<b>413</b>	<b>1,285</b>	<b>321</b>	<b>595</b>	<b>2,614</b>
<b>At December 31, 2013</b>					
Cost	1,264	1,285	3,134	1,383	7,066
Accumulated depreciation	(851)	—	(2,813)	(788)	(4,452)
<b>Net book amount</b>	<b>413</b>	<b>1,285</b>	<b>321</b>	<b>595</b>	<b>2,614</b>
<b>As of January 1, 2014</b>					
<b>Opening net book amount</b>	<b>413</b>	<b>1,285</b>	<b>321</b>	<b>595</b>	<b>2,614</b>
Reclassifications	12,543	(15,355)	2,149	663	—
Additions	10	14,489	1,849	443	16,791
Depreciation charge	(804)	—	(218)	(517)	(1,539)
Currency translation effects	1,220	465	(20)	136	1,801
<b>Closing net book amount</b>	<b>13,382</b>	<b>884</b>	<b>4,081</b>	<b>1,320</b>	<b>19,667</b>
<b>At December 31, 2014</b>					
Cost	15,074	884	7,200	2,544	25,702
Accumulated depreciation	(1,692)	—	(3,119)	(1,224)	(6,035)
<b>Net book amount</b>	<b>13,382</b>	<b>884</b>	<b>4,081</b>	<b>1,320</b>	<b>19,667</b>

Construction in Process ("CIP") at December 31, 2013 and December 31, 2014 related to the build-out of the manufacturing facility in Lexington, Massachusetts, that had started at the end of the second quarter of 2013.

Total depreciation expense of €1,539,000 for the twelve months ended December 31, 2014 (twelve months ended December 31, 2013: €535,000, 2012: € 548,000) has been charged to research and development expense where it relates to our manufacturing facility and equipment, and to selling, general and administrative expense for other matters.

### 7. Other Non-Current Assets

As of December 31, 2014, the amount represents a refundable security deposit for the lease payments of the Lexington, Massachusetts facility, paid in September 2013, accrued with Interest on the balance sheet.



## UNIQUE N.V.

### Notes to Consolidated Financial Statements (Continued)

For the Years Ended December 31, 2012, 2013 and 2014

#### 8. Trade and Other Receivables

	DECEMBER 31, 2013	DECEMBER 31, 2014
	(€ in thousands)	
Receivables from related parties	1,425	2,426
Other receivables	764	588
Prepaid Expenses	391	515
Social security and other taxes	402	439
<b>Trade and other receivables</b>	<b>2,982</b>	<b>3,968</b>

The fair value of trade and other receivables approximates their carrying value. As of December 31, 2014 and December 31, 2013, all trade and other receivables were assessed as fully recoverable. The carrying amount of the Company's trade receivables are denominated in Euro and USD.

The receivables from related parties as of December 31, 2014 relate to amounts due from Chiesi based on revenue recognized and expenses reimbursed of €2,404,000; (2013: €1,402,000). The remaining element of receivables from related parties relate to certain wage tax liabilities settled by the Company 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 of €22,000; (2013: 22,000).

The Other Receivables balance at December 31, 2014 consists of certain deposits made in relation to the further build-out of the US facility and accrued income in relation to grants. The Other Receivables balance at December 31, 2013 consists largely of amounts of tenant improvements due to the Company from the landlord in relation to our facility in Lexington, Massachusetts (€546,000), as well as prepayments related to rent, insurance and certain annual license fees in software and intellectual property.

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.

#### 9. Business Combinations

On July 15, 2014 the Company signed and on July 31, 2014 the Company closed an agreement to acquire all shares of InoCard GmbH. InoCard was founded in December 2013 as a spin-off of the University of Heidelberg, and is an early-stage biotechnology company focused on the development of gene therapy approaches for cardiac disease. InoCard has developed a novel gene therapy through preclinical proof of concept, for the one-time treatment of congestive heart failure (CHF). InoCard founders Prof. Patrick Most and Prof. Hugo Katus have joined uniQure as Managing Director of uniQure in Germany and Chairman of the Scientific Advisory Board, for Cardiovascular Diseases, respectively.

Under the terms of the agreement, InoCard shareholders have received an upfront payment of approximately €3,000,000 (€1,500,000 in cash and €1,500,000 in uniQure shares (189,982 shares at closing of the transaction)), and will receive a further €14,500,000 in success-based milestone payments

**UNIQUE N.V.**

## Notes to Consolidated Financial Statements (Continued)

For the Years Ended December 31, 2012, 2013 and 2014

**9. Business Combinations (Continued)**

upon achieving certain clinical and regulatory targets. Upon a successful commercial launch of a developed product, the sellers will further receive a royalty payment of 0.5% of the net product sales. The amount of the €14,500,000 in milestones is payable, at the Company's sole discretion, in either cash or a variable number of Company shares, based on the then current stock price.

The acquired entity, InoCard, is effectively a single-product business, fully focusing on the further development of gene therapy approaches for cardiac disease. All success based milestones relate to the further development of these programs and therefore these programs are deemed the only material asset of the entity. As such, the value of InoCard is assumed to fully be represented by the fair value of the S100A1 program. As of the acquisition date the Company performed a purchase price allocation under IFRS 3 that resulted in a fair value assessment of the acquired IPR&D asset in a value of €4,665,000.

In determining the fair value of IPR&D, the Company utilized the Income Approach (Discounted Cash Flow method). Inputs to this model were assumptions on pricing and market share developments, together with assumptions on the cumulative probability of success of progressing through the various clinical development stages up to market approval; This method resulted in a series of future cash flow that were discounted at a rate of 30%.

The following table summarises the consideration paid for InoCard and the amounts of the assets acquired and liabilities assumed, recognized at the acquisition date:

	<u>July 31, 2014</u> (€ in thousands)
<b>Consideration paid:</b>	
Cash paid	1,463
Shares	1,500
Shares issued upon conversion of assumed convertible loan	17
Contingent consideration	1,301
<b>Total consideration</b>	<b><u>4,281</u></b>

The closing share price on July 31, 2014 was \$10.22

## UNIQUE N.V.

### Notes to Consolidated Financial Statements (Continued)

For the Years Ended December 31, 2012, 2013 and 2014

#### 9. Business Combinations (Continued)

Recognized amounts of identifiable assets acquired and liabilities assumed were as follows:

	<u>July 31, 2014</u> (€ in thousands)
<b>Non-current assets</b>	
Intangible assets (excl. Goodwill)	4,665
<b>Current assets</b>	
Cash and cash equivalents	373
VAT receivable	13
<b>Non-current liabilities</b>	
Deferred tax liabilities	(1,379)
<b>Current liabilities</b>	
Trade payables	(7)
Other payables	(726)
<b>Total identifiable net assets</b>	<u><u>2,939</u></u>
<b>Goodwill</b>	<u><u>1,342</u></u>

In relation to this acquisition an amount of €258,000 was recognized as transaction cost in the Selling, general and administrative expenses for the year ended December 31, 2014.

The fair value of the contingent consideration is estimated as the expected (i.e. probability-weighted) present value of the milestone payments and based on a discount rate of 30%. The relatively high discount rate is derived from the high uncertainty of progressing from the current pre-clinical development stage through the various clinical stages before arriving at a commercial stage. The fair value of this contingent consideration will be re-measured every reporting date with changes recognized in profit & loss for the period. The fair value could change as the timing or the probability of achieving the milestone payments changes, or due to the time value of money. The contingent consideration calculated at initial recognitions as €1,301,000 is accounted for as a liability. The maximum, undiscounted contingent consideration amounts to €14,500,000 upon achieving clinical milestones with an additional 0.5% royalty of future net product sales.

At reporting date the updated valuation of the contingent consideration resulted in an additional liability of €153,000 that was subsequently taken as research and development expense through the profit and loss accounts of the Company.

This classification was determined on the basis that the movements in fair value should follow the nature and purpose of the contingent consideration, arising from achieving operational milestones in the further development of the underlying product. The fair value of the contingent consideration at December 31, 2014 is €1,454,000.

The IPR&D is not recognized for tax purposes; therefore a deferred tax liability is recognized for this temporary difference. The deferred tax liability is based on the fair value of the IPR&D multiplied by the German tax rate of 29.58%, resulting in a deferred tax liability of €1,379,000.

# UNIQUE N.V.

## Notes to Consolidated Financial Statements (Continued)

For the Years Ended December 31, 2012, 2013 and 2014

### 9. Business Combinations (Continued)

The operational loss included in the consolidated statement of comprehensive loss from August 1, 2014 to December 31, 2014 contributed by InoCard GmbH was €400,000. No revenues were contributed by InoCard.

Had InoCard been consolidated from January 1, 2014, the consolidated income statement for the twelve months ended December 31, 2014 would show a pro-forma revenue of €0 and a pro-forma loss of €763,000.

### 10. Inventories

	DECEMBER 31, 2013	DECEMBER 31, 2014
	(€ in thousands)	
Raw materials	103	152
Work in process / Intermediate Products	762	48
<b>Inventories</b>	<b>865</b>	<b>200</b>

Inventories as of December 31, 2014 were €200,000 (2013:€865,000). 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 early 2015. Also included in inventories are amounts assigned to commercial batches of Glybera. The reduction in the inventories over the course of 2014 related to a number of batches, manufactured in 2013, that were in 2014 considered to be out of specifications and could not be put forward for commercial sale; the net reduction in 2014 of €714,000 (2013: nil) was accordingly booked into research and development expenses.

### 11. Cash and Cash Equivalents

	DECEMBER 31, 2013	DECEMBER 31, 2014
	(€ in thousands)	
Cash at bank and in hand	23,810	53,219
	<u>23,810</u>	<u>53,219</u>

The cash balance as of December 31, 2014 reflects the balance of our expenses and investments, and the proceeds from the IPO for €62.0 million after commissions and expenses and from the amendment to the venture debt financing from Hercules Technology Growth Capital for \$9.8 million (€7.2 million).

### Supplemental information relating to the Cash Flow Statement

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 December 31, 2013. Refer to Note 15 below.

# UNIQUE N.V.

## Notes to Consolidated Financial Statements (Continued)

For the Years Ended December 31, 2012, 2013 and 2014

### 11. Cash and Cash Equivalents (Continued)

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 €2,113,000, represented non-cash items as of December 31, 2013.

Purchases of fixed assets and changes in trade and other payables exclude a non-cash item of €1,022,000 largely related to the purchase of fixed assets, which have not yet been paid as of December 31, 2014. (2013: €628,000 and 2012: nil)

All non-cash items described above are excluded from the Consolidated Statement of Cash Flows on page F-6.

### 12. Shareholders' (Deficit)/Equity

uniQure was incorporated on January 10, 2012; therefore, the year ended December 31, 2012 is the first accounting period for the Company.

	NUMBER OF SHARES	AMOUNT OF UNIQUE CAPITAL (BASED ON SHARES OF €0.05 NOMINAL VALUE) (€ in thousands)
<b>Share capital (ordinary shares)</b>		
<b>As of December 31, 2012</b>	<b>9,653,495</b>	
Share capital		483
Share premium		114,795
<b>Total</b>		<b>115,278</b>
<b>New shares issued in 2013</b>	<b>2,541,411</b>	
Share capital		127
Share premium		27,664
<b>Total</b>		<b>27,791</b>
<b>As of December 31, 2013</b>	<b>12,194,906</b>	
Share capital		610
Share premium		142,459
<b>Total</b>		<b>143,069</b>
<b>New shares issued in 2014</b>	<b>5,897,288</b>	
Share capital		295
share premium		64,320
<b>Total</b>		<b>64,615</b>
<b>As of December 31, 2014</b>	<b>18,092,194</b>	
Share capital		905
Share premium		206,111
<b>Total</b>		<b>207,016</b>

# UNIQUE N.V.

## Notes to Consolidated Financial Statements (Continued)

For the Years Ended December 31, 2012, 2013 and 2014

### 12. Shareholders' (Deficit)/Equity (Continued)

On January 31, 2014, we effected a 5-for-1 consolidation of our shares, which had the effect of a reverse share split. All share, per-share and related information presented in these financial statements has been retroactively adjusted, where applicable, to reflect the impact of this reverse share split.

As of the date hereof, our authorized share capital is €3,000,000, divided into 60,000,000 ordinary 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.

Following the IPO where the Company issued 5,400,000 ordinary shares, and as of December 31, 2014, a total of 18,092,194 shares were issued and paid up in full at a nominal value of €0.05 per share (December 31, 2013: 12,194,906 shares at €0.05 per share, December 31, 2012: 9,653,495 shares at €0.05 per share). Of these 18,092,194 shares, a total of 5,897,288, are presented as being issued during the year (2013: 2,541,411, 2012: 4,902,473 shares. The total gross payment with respect to these shares issued during the period is presented as €64,615,000 (2013: €27,791,000, 2012: €15,094,000).

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 were 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 required the approval of the general meeting of the class A ordinary shares, class A, B and C ordinary shares carried equal economic rights and ranked equally. As per the IPO date the Company reclassified all the Class A, B and C ordinary shares as one single category of ordinary shares.

Date	Description	Number of Shares	Share Capital Amounts	Share Premium Amounts	Total Equity Amounts
				(€ in thousands)	
January 1, 2013	Brought forward	9,653,496	483	114,795	115,278
January - May, 2013	Employee and other persons new equity investments	90,747	4	274	278
July 24, 2013	Chiesi new equity investment	1,109,214	55	13,945	14,000
July 26, 2013	Conversion of 2012 & 2013 convertible loan	1,336,331	67	13,430	13,497
November 2013	Exercise of options	5,118	1	15	16
February 05, 2014	Initial Public Offering	5,400,000	270	61,683	61,953
July 31, 2014	Issuance of shares	192,128	10	1,507	1,517
September - December 2014	Exercise of options	305,160	15	462	477
<b>December 31, 2014</b>		<b>18,092,194</b>	<b>905</b>	<b>206,111</b>	<b>207,016</b>

This note describes the shares issued during the period since January 1, 2013. In summary these were as follows:

- In January 2013 pursuant to an agreement entered into in April 2012, the Company completed raising (that started in late 2012 with a total amount of €274,000 through the issuance of an aggregate of 89,155 class B ordinary shares) with a further amount of €278,000 through the issuance of an aggregate of 90,747 class B ordinary shares, represented by uniQure DRs shares to employees and related parties at a price of € 3.07 per share;
- On July 24, 2013 pursuant to various agreements with Chiesi Pharmaceutici S.p.A the Company raised a total amount of €14,000,000 through the issuance of 1,109,214 Class C ordinary shares at a price of €12.60 per share;

**UNIQUE N.V.**

## Notes to Consolidated Financial Statements (Continued)

For the Years Ended December 31, 2012, 2013 and 2014

**12. Shareholders' (Deficit)/Equity (Continued)**

- On July 26, 2013 the Company converted the 2012 Convertible loan through the issuance of 1,336,333 Class A shares at a price of €10.10 per share; and
- In November 2013 through conversion of share options the Company issued 5,118 Class B ordinary shares at a price of €3.07 per share.
- On February 5, 2014 pursuant to the Initial Public Offering the Company issued 5,400,000 ordinary shares at a nominal value of €0.05 per share, generating €61,953,000 (after commission and expenses).
- On July 31, 2014 pursuant to the acquisition of InoCard GmbH the Company issued 192,128 ordinary shares at a nominal value of €0.05 per share.
- From September 2014 through to December 31, 2014 through exercise of share options the Company issued 305,160 ordinary shares at a price ranging from €0.05 - €3.07 per share.

In November 2013 a total of 5,118 shares were issued upon exercise of share options. In 2014 a total of 305,160 shares were issued upon exercise of options.

As of December 31, 2014, 7,258 shares were held by the stichting participatie AMT as treasury shares (2013: 7,258). (Further details of stichting participatie AMT are set out in Note 1 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, 2014 amount to €63,652,000 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.

The Company, in 2013, presented in other reserves the result of the conversion of the convertible loan to the amount of €3,005,000 (see Note 15). In addition, in 2014 the Company presents under Other Reserves, Other Comprehensive Income arising from the foreign currency translation difference from the U.S. subsidiary.

In the years presented in these financial statements, the Company did not have any other legal or other types of restricted reserves.

**UNIQURE N.V.**

## Notes to Consolidated Financial Statements (Continued)

For the Years Ended December 31, 2012, 2013 and 2014

**12. Shareholders' (Deficit)/Equity (Continued)**

No tax amounts are included under other comprehensive income as the Company does not record any income tax expense.

**13. Share Based Payments****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 were granted on the date of grant and vest over a period of three years on the basis set out in Note 2.4.18 above. Any options that vest must be exercised by the tenth anniversary of the effective date of grant.

In 2012 a total of 1,606,347 options were granted under the 2012 Plan to management and certain other employees and consultants. The expense recognized amounted to €1,767,000 during the year ended December 31, 2012. In the year 2013 the Company granted another 301,468 options, a total of 210,853 were forfeited and a total of 5,118 options were exercised, to result in an ending balance as of December 31, 2013 of a total of 1,691,844 outstanding options recognizing a share based expense of €2,023,000.

**2014 Share Option Plan**

At the general meeting of shareholders on January 9, 2014, uniQure shareholders approved the adoption of the 2014 Incentive Plan. Under the 2014 Incentive Plan, share options were granted on the date of grant and vest over a period of four years on the basis set out in Note 2.4.18 above. Any options that vest must be exercised by the tenth anniversary of the effective date of grant.

**4D Option Plan**

Following the agreement between the Company and 4D Molecular Therapeutics, the Company has granted options to purchase an aggregate of 609,744 ordinary shares in connection with this collaboration, and will recognize resulting share-based payment expense over three years (4D Option Plan).

In the year 2014, for the above mentioned plans, the Company granted a total of 1,724,744 options, of which 57,588 were forfeited and 305,160 options were exercised, at a weighted average share price at exercise date of \$14.36 (€11.56), resulting in an ending balance as of December 31, 2014 of 3,053,840 outstanding options and the recognition of a share based expense of € 9,114,000.

**Restricted Stock Units (RSU)**

In the year 2014 the Company granted a number of 179,068 RSU's for which the Company recognized a share based expense of €350,000. Inputs to the valuation of the granted RSU's, are the share price at date of grant and the anticipated date of full and final vesting. The fair value at grant of the RSU's is determined at \$1.8 million (€1.5 million).



# UNIQUE N.V.

## Notes to Consolidated Financial Statements (Continued)

For the Years Ended December 31, 2012, 2013 and 2014

### 13. Share Based Payments (Continued)

The 2012 Option Plan, the 2014 Option Plan and the 4D Option plan all qualify as equity-settled option plans. Movements in the number of outstanding share options granted in 2012, 2013 and 2014, under all Plans, were as follows:

	2012		2013		2014	
	NUMBER	EXERCISE PRICE	NUMBER	EXERCISE PRICE	NUMBER	EXERCISE PRICE
Options outstanding as of January 1	379,640	€9.75 - €14.60	1,606,347	€3.07	1,691,844	€3.07 - €10.10
Options granted	1,606,347	€3.07	301,468	€3.07 - 10.10	1,724,744	€0.05 and \$9.35 - \$9.63
Options forfeited	(379,640)	€9.75 - €14.60	(210,853)	€3.07	(57,588)	€3.07 and \$9.35
Options exercised	—	—	(5,118)	€3.07	(305,160)	€0.05 - €3.07
Options outstanding as of December 31	<u>1,606,347</u>	<u>€3.07</u>	<u>1,691,844</u>	<u>€3.07 - €10.10</u>	<u>3,053,840</u>	<u>€0.05 - €10.10 and \$9.35 - \$9.63</u>

Of the 3,053,840 options outstanding (2013: 1,691,844, 2012: 1,606,347), 1,423,175 options (2013: 773,442, 2012: nil) were vested and exercisable (within limitations of the Company's Insider Trading Policy). 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, 2014 WEIGHTED AVERAGE REMAINING CONTRACTUAL LIFE	RANGE EXERCISE PRICE PER SHARE	NUMBER OF OPTIONS
1 - 5 years	€0.05	457,308
6 years		
7 years	€3.07	1,435,653
8 years	€10.10	92,129
9 years	\$9.35 - \$9.63	1,068,750
At December 31, 2014		<u>3,053,840</u>

YEAR ENDED DECEMBER 31, 2013 WEIGHTED AVERAGE REMAINING CONTRACTUAL LIFE	RANGE EXERCISE PRICE IN EUR PER SHARE	NUMBER OF OPTIONS
1 - 5 years	—	—
6 years	—	—
7 years	—	—
8 years	3.07	1,397,127
9 years	3.07 - 10.10	294,717
At December 31, 2013	3.07 - 10.10	<u>1,691,844</u>

# UNIQUE N.V.

## Notes to Consolidated Financial Statements (Continued)

For the Years Ended December 31, 2012, 2013 and 2014

### 13. Share Based Payments (Continued)

YEAR ENDED DECEMBER 31, 2012 WEIGHTED AVERAGE REMAINING CONTRACTUAL LIFE	RANGE EXERCISE PRICE IN € PER SHARE	NUMBER OF OPTIONS
1 - 5 years	—	—
6 years	—	—
7 years	—	—
8 years	—	—
9 years	3.07	1,606,347
At December 31, 2012	3.07	1,606,347

The Black-Scholes option pricing model has been used to value these awards, based on the following key variables:

	2012	2013	2014
Options with change of control and service based vesting conditions	—	—	3,053,840
Options with an IPO, change of control and service based vesting conditions	1,606,347	1,691,844	—
Share Price: the closing share price on the grant dates	—	—	\$8.66 - 9.63
Estimated fair value per option as of grant date	€2.05 - 3.60	€3.40 - 12.35	\$5.24 - 5.93
Expected Volatility: uniQure used an estimated volatility figure which was determined based on volatility analysis of companies in the same sector and of a similar size	70 - 80%	70%	70%
Expected Term: is the period from grant until the expected exercise date.	5.5 - 6.3 years	5.5 - 6.3 years	6.11 years
Exercise price:	€3.07	€3.07 - 10.10	€0.05 and \$9.35 - 9.63
Expected Dividend Yield: the Company currently does not pay dividends and has no plans to do so	0%	0%	0%
Risk-free Rate: based on Government bonds with a term that is 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%	0.4 - 1.2%	0.23%

Of the 1,606,347 options granted in 2012, 478,217 options were granted to members of the Management Board and 196,912 options were granted to members of the Supervisory Board. In 2013, 301,468 options were granted (of which 252,652 options were granted to members of the Management Board and 10,000 options were granted to a member of the Supervisory Board). A total of 210,853

**UNIQURE N.V.**

## Notes to Consolidated Financial Statements (Continued)

For the Years Ended December 31, 2012, 2013 and 2014

**13. Share Based Payments (Continued)**

options were forfeited in 2013 (of which 140,652 options were forfeited from a member of the Management Board and 37,507 options were forfeited from a member of the Supervisory Board). In November 2013, a total of 5,118 options were exercised.

Under the 2014 Option Plan a total of 1,115,000 options were granted (of which 332,500 options were granted to members of the Management Board and Senior Management). None of these options granted to the Management Board and Senior Management lapsed / forfeited during 2014. Another 35,000 options were granted to members of the Supervisory Board of which a total of 5,000 lapsed / forfeited in 2014. Of the remaining 747,500 options granted in 2014, 41,250 lapsed / forfeited in 2014. As of December 31, 2014 there were 3,053,840 options outstanding. An additional €4.8 million of share - based expense is expected to be recognized from 2015 through to 2018.

**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 limited, 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 midpoint between the vesting date and the contractual term of the options.

**Valuation of ordinary shares**

The Company's shares are listed on the NASDAQ (ticker: QURE). At the date of each grant of options subsequent to the transaction between uniQure and AMT, and prior to date of listing, 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**

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, an applied volatility of 70% in respect of the options granted in the year ended December 31, 2013 and an applied volatility of 70% in respect of the options granted in the year ended December 31, 2014. Based on the limited trading history of the Company's shares on NASDAQ, between February 5, 2014 and December 31, 2014 the volatility is calculated at 71.9%

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 30. The corresponding increase in equity is separately accounted for as other reserves.

# UNIQURE N.V.

## Notes to Consolidated Financial Statements (Continued)

For the Years Ended December 31, 2012, 2013 and 2014

### 14. 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 wholly-owned subsidiary of the AMC, and uniQure, regarding leasehold improvements at Meibergdreef, Amsterdam, ended at September 30, 2016. The rent of the leasehold improvements amounts to €156,000 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.

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 5.5% per annum.

	DECEMBER 31, 2013	DECEMBER 31, 2014
	(€ in thousands)	
Gross finance lease liabilities—minimum lease payments		
No later than 1 year	184	184
Later than 1 year and no later than 5 years	322	138
Later than 5 years	—	—
Future finance charges on finance leases	(48)	(20)
<b>Total</b>	<b>458</b>	<b>302</b>

The present value of finance lease liabilities is as follows:

	DECEMBER 31, 2013	DECEMBER 31, 2014
	(€ in thousands)	
No later than 1 year	156	168
Later than 1 year and no later than 5 years	302	134
Later than 5 years	—	—
Future finance charges on finance leases	—	—
<b>Total</b>	<b>458</b>	<b>302</b>

### 15. Debt to related party

#### December 2012 Convertible loan and amendment in March 2013 / Conversion in July 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.

**UNIQUE N.V.****Notes to Consolidated Financial Statements (Continued)**

For the Years Ended December 31, 2012, 2013 and 2014

**15. Debt to related party (Continued)**

In March 2013, uniQure increased the loan by an additional €10,000,000 investment by Collier Capital. As part of the increase, the loan note terms for all loan note holders described in the annual consolidated financial statements were amended such that the final maturity date of the loan notes was extended to December 31, 2014. Additionally, the warrant entitlement was reduced to 10% of the principal amount of the loan provided to uniQure.

Following the subscription for new equity by Chiesi, on July 21, 2013 the full convertible loan of €13,497,000 was converted on July 26, 2013 into new Class A Ordinary Shares, at a conversion price of €10.10 per share. This conversion marked the extinguishment of the convertible derivative instrument. The remaining derivative element relates to the warrants issued to the holders of the convertible loan as part of the convertible loan arrangements.

The warrants associated with the convertible loan, which survived the conversion of the loan, are presented in the consolidated balance sheet as at December 31, 2014 within liabilities as an embedded derivative with a fair value of €645,000 (December 31, 2013: €722,000).

Where it comes to debts to related parties, during the period ended December 31, 2014, an amount of € 77,000 was recognized as finance income (compared with a loss of €4,387,000 for period ended December 31, 2013). The 2014 amount related to the surviving warrants and the 2013 amount related to €3,491,000 of derivative result on conversion where the remainder consisted of interest expense in relation to the convertible note. The elimination of the embedded derivative (convertible element) by the early conversion of the loan in July 2013 created €3,005,000 of Other Reserves within the Equity presentation.

**16. Trade and Other Payables**

	<b>DECEMBER 31, 2013</b>	<b>DECEMBER 31, 2014</b>
	<b>(€ in thousands)</b>	
Trade payables	3,507	4,860
Social security and other taxes	802	963
Other current liabilities	3,292	3,794
<b>Total trade and other payables</b>	<b><u>7,601</u></b>	<b><u>9,617</u></b>

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

**Other current liabilities**

As of December 31, 2014 and December 31, 2013, other current liabilities consisted principally of accruals for services provided by vendors but not yet billed, reimbursements received from research and development partners for expenses which have yet to be incurred and miscellaneous liabilities.

# UNIQUE N.V.

## Notes to Consolidated Financial Statements (Continued)

For the Years Ended December 31, 2012, 2013 and 2014

### 17. Borrowings

	DECEMBER 31, 2013	DECEMBER 31, 2014
	(€ in thousands)	
<b>Non-current</b>		
Borrowings	6,292	16,418
<b>Total non-current</b>	<b>6,292</b>	<b>16,418</b>
<b>Current</b>		
Debt to related party—derivative	722	645
Borrowings	633	—
Borrowings—derivative	217	207
<b>Total current</b>	<b>1,572</b>	<b>852</b>
<b>Total</b>	<b>7,864</b>	<b>17,270</b>

### 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 €7,062,000 as of June 30, 2014, presented net of expenses for facility charges of 1.25% plus expenses related to legal counsel. The loan commitment is \$10,000,000 with an interest rate of 11.85% or an amount equal to 11.85% plus the prime rate of interest minus 3.25%, 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).

During 2014, an amount of \$2.0 million (€1.6 million), compared with \$0.7 million (€0.5 million) for 2013, was recorded as finance expense in relation to the Hercules borrowing.

The warrant included in this loan agreement is not closely related to the host contract and therefore has been split and accounted for separately as a financial derivative measured at fair value through profit or loss. The fair value of this derivative is €207,000 (2013: 217,000) and is included within the current liabilities: Borrowings—derivative on the Consolidated Balance Sheet as of December 31, 2014.

On June 26, 2014 the Company entered into an amended and restated loan agreement (which amends and replaces the original loan agreement) of \$20,000,000 (then €14,600,000), presented net of expenses for facility charges of 1.00% plus expenses related to legal counsel. The additional amount of \$10,000,000 (€7,344,000) was received net of expenses of \$218,000 (€160,000). The net cash inflow was \$9,782,000 (€7,184,000). The total loan commitment is \$20,000,000 with an interest rate of 10.25% which matures over a period of 48 months. Also included are two back-end fees of \$345,000 and \$250,000, due October 2016 and June 2018 respectively. The interest-only period is 18 months. We are required to repay the loan in monthly principal installments from January 2016 through June 2018. As the terms of the amended loan agreement changed significantly compared to the original loan agreement (maturity date, interest rate, payback schedule), the Company fully amortized the

# UNIQURE N.V.

## Notes to Consolidated Financial Statements (Continued)

For the Years Ended December 31, 2012, 2013 and 2014

### 17. Borrowings (Continued)

unamortized transaction costs at issue, resulting in an extra amortisation charge through profit and loss in 2014 of \$193,000 (€141,000).

The total value for the amended loan per December 31, 2014 was \$20.0 million (€16.4 million) and is recorded net of expenses under non-current borrowings. The warrants included in the original loan agreement remain in place and are unaffected. The fair value of the borrowings equals their carrying amount, as the impact of discounting is insignificant as the loan is already amortized at a market conform interest rate.

The foreign exchange expense on the borrowings was €1.8 million in 2014. In the period ended December 31, 2014 the current element of this loan facility reduced to nil, as the amended agreement introduced a further extension of the interest only period.

The amended 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 have periodic reporting requirements and 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. This restriction on the cash reserves only relates to the location of the cash reserves, but all cash reserves are at free disposal of the Company. The amended Loan and Security Agreement contains default provisions that include the occurrence of a material adverse effect, as defined therein, which would entitle Hercules to declare all principal, interest and other amounts owed by us immediately due and payable. As of December 31, 2014, we were in compliance with these covenants in all material respects.

### 18. Revenues and Deferred Revenues

	FOR THE YEARS ENDED		
	DECEMBER 31, 2012	DECEMBER 31, 2013	DECEMBER 31, 2014
		(€ in thousands)	
License revenues	—	440	883
Collaboration revenues	—	2,503	3,802
<b>Total</b>	<b>—</b>	<b>2,943</b>	<b>4,685</b>

	DECEMBER, 31 2013	DECEMBER, 31 2014
	(€ in thousands)	
Deferred revenues current portion	1,279	1,328
Deferred revenues	15,679	15,387
<b>Total</b>	<b>16,958</b>	<b>16,715</b>

During the period ended December 31, 2014, an amount of €883,000 (period ended December 31, 2013: €440,000, December 31, 2012: € nil) was recognized as license revenues. This amount relates to

**UNIQURE N.V.**

## Notes to Consolidated Financial Statements (Continued)

For the Years Ended December 31, 2012, 2013 and 2014

**18. Revenues and Deferred Revenues (Continued)**

the recognition of the up-front payments received from Chiesi. During the period ended December 31, 2014, an amount of €3,802,000 (period ended December 31, 2013: €2,503,000) 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 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; and
- 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 file for regulatory approval in late 2019. 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 18 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 a remaining 18 years.

For the period ended December 31, 2013, the Company recognized an expense, under cost 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 29, Contingent Liabilities.



**UNIQURE N.V.**

## Notes to Consolidated Financial Statements (Continued)

For the Years Ended December 31, 2012, 2013 and 2014

**18. Revenues and Deferred Revenues (Continued)**

Collaboration revenues from contracts, typically from delivering research and development services, relate to the agreements, and are dependent upon the nature of the invoice either recognized on the basis of labor hours delivered at the Agreements' full time employee rate, based on an agreed allocation key of certain expenses.

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.

**19. Other Income/Other Losses**

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 €773,000 in 2014 (2013: €585,000, 2012 €649,000) and relates to grants received and rebates on payroll taxes. In 2014 uniQure, Inc., our wholly owned subsidiary, received a grant from Massachusetts Life Science Company under its Job Incentive Program (New Job Creation). The monthly amortization (€13,000) of this grant started in December 2014.

The other gains / losses line represents the currency effect from regular operations whereas the currency risk associated with borrowings is presented under Finance Income or Expense.

**20. Expenses by Nature**

Research and development costs amounted to €33,932,000 €13,182,000 and €10,231,000 in 2014, 2013 and 2012 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 €11,167,000, €11,628,000 and €4,564,000 in 2014, 2013 and 2012, respectively, and consist of allocated

# UNIQUE N.V.

## Notes to Consolidated Financial Statements (Continued)

For the Years Ended December 31, 2012, 2013 and 2014

### 20. Expenses by Nature (Continued)

employee costs, office costs, consultancy costs and administrative costs. Research and development costs and general administrative costs included the following costs by function:

	YEARS ENDED DECEMBER 31,		
	2012	2013	2014
	(€ in thousands)		
Employee benefit expenses	8,350	11,904	25,349
Laboratory and development expenses	2,065	3,404	5,462
Legal and advisory expenses	1,622	5,001	5,779
Office and housing expenses	1,197	1,592	3,776
Patents and licenses	619	835	744
Other operating expenses	394	1,539	2,450
Depreciation expenses (See note 6)	548	535	1,539
Other losses/(gains)—net (exchange differences)	45	453	(5,807)
	<u>14,840</u>	<u>25,263</u>	<u>39,292</u>

Under Employee benefit expenses for the year ended December 31, 2014 the Company recorded share-based payments of €9.5 million, of which €6.3 million is related to the 4D Option Plan. Share-based payments, recorded under Employee benefit expenses, for the years ended December 31, 2012 and 2013 were €1,508,000 and €2,023,000 respectively.

### 21. Research and development expenses

Research and development expenses increased from €13,182,000 in the period ended December 31, 2013 to €33,932,000 in the period ended December 31, 2014. This increase reflected the expansion of our research and development activities to support the pre-clinical activities and planned clinical study of AMT-060, the planned commercial launch of Glybera in the European Union, the build-up of staff in our Lexington facility, as well as the further development of Glybera and our other product candidates. In addition, as part of our strategic and license collaboration with 4D Molecular Therapeutics entered into in January 2014, we incurred increased research and development expenses related to certain stock-based payments made to 4D Molecular Therapeutics.

Research and development expenses increased from €10,231,000 in the period ended December 31, 2012 to €13,182,000 in the period ended December 2013, 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.

### 22. General and administrative expenses

General and administrative expenses decreased from €11,628,000 for the period ended December 31, 2013 to €11,167,000 for the period ended December 31, 2014. This decrease resulted principally from the high legal and audit related expenses incurred in 2013 for the preparation of our initial public offering, partially offset by an increase of expenses in the period ended December 31, 2014, related to being a public company, and the continued build-out of the administrative functions.

**UNIQUE N.V.**

## Notes to Consolidated Financial Statements (Continued)

For the Years Ended December 31, 2012, 2013 and 2014

**22. General and administrative expenses (Continued)**

General and administrative expenses increased from €4,564,000 for the period ended December 31, 2012 to €11,628,000 for the period ended December 31, 2013. The increase is primarily due to expenses related to consultants (commercial, operations and administrative) and professional fees.

**23. Other Comprehensive Income**

For the period ended December 31, 2014 other comprehensive income of €1,149,000 represents the foreign currency translation arising from the U.S. subsidiary, which was established in 2013 (for the period ended December 31, 2013: €12,000, 2012: € nil).

**24. Employee Benefit Expense**

	YEARS ENDED DECEMBER 31,		
	2012	2013	2014
	(€ in thousands)		
Wages and salaries	4,553	5,012	9,888
Social security costs	361	377	900
Share-based payments (option plans and RSUs)	1,767	2,023	9,464
Pension costs—defined contribution plans	303	415	610
Other employee expenses	1,366	4,077	4,487
	<b>8,350</b>	<b>11,904</b>	<b>25,349</b>
Number of employees at the end of the period	67	87	162

For detailed disclosure on the remuneration of the Supervisory Board, the Management Board and Senior Management please refer to note 31.

# UNIQUE N.V.

## Notes to Consolidated Financial Statements (Continued)

For the Years Ended December 31, 2012, 2013 and 2014

### 25. Finance Income and Expense

	YEARS ENDED DECEMBER 31,		
	2012	2013	2014
	(€ in thousands)		
Finance income:			
Interest income current accounts	22	58	167
Derivative result	—	44	87
	<u>22</u>	<u>102</u>	<u>254</u>
Finance expense:			
Derivative result arising on early conversion of the loan	(464)	(1,333)	—
Derivative result	—	(2,158)	—
Loan from related party	(63)	(691)	—
Venture debt facility	—	(165)	(3,432)
Finance leases	(20)	(40)	(28)
	<u>(547)</u>	<u>(4,387)</u>	<u>(3,460)</u>
Finance costs—net	<u>(525)</u>	<u>(4,285)</u>	<u>(3,206)</u>

The amount presented for the venture debt facility for the period ended December 31, 2014 consists of an amount of €1,598,000 of interest, where the balance of €1,834,000 is the foreign exchange result on the loan.

# UNIQUE N.V.

## Notes to Consolidated Financial Statements (Continued)

For the Years Ended December 31, 2012, 2013 and 2014

### 26. Income Tax Expense

In the Netherlands no tax charges or liabilities were incurred in 2012, 2013 and 2014 since the Company was in a loss-making position. No deferred tax asset has been recognized in respect of carry-forward losses.

	YEARS ENDED DECEMBER 31,		
	2012	2013	2014
	(€ in thousands)		
<b>Netherlands</b>			
Current tax	—	—	—
Deferred tax	—	—	—
Profit/(loss) before tax	(14,716)	(26,222)	(28,214)
Expenses not deductible for tax purposes in the Netherlands	2,318	5,123	9,590
Tax losses for which no deferred tax asset was recognized in the Netherlands	(12,398)	(21,099)	(18,624)
Tax charge	—	—	—
<b>Germany</b>			
Current tax	—	—	—
Deferred tax	—	—	1,379
Profit/(loss) before tax	—	—	(247)
Expenses not deductible for tax purposes	—	—	—
Tax losses for which no deferred tax asset was recognized	—	—	(247)
Tax charge	—	—	—
<b>United States</b>			
Current tax	—	—	—
Deferred tax	—	—	—
Profit/(loss) before tax	—	(585)	(7,430)
Expenses not deductible for tax purposes	—	585	3,273
Tax losses for which no deferred tax asset was recognized	—	—	(4,157)
Tax charge	—	—	—

The amount presented for the 2014 tax loss under the Dutch tax regime, is a pro-forma calculation, reconciling the Company's commercial loss to an estimated tax loss. The expenses not deductible for tax purposes are largely driven by the sum of the Company's share based expenses, differences in timing and duration of depreciation on certain tangible assets. The pro-forma amount previously presented for 2013 changed from €(24,659,000) to €(21,099,000) following a further assessment by the Dutch tax authorities.

Following the InoCard transaction in Germany in 2014 the Company has recognized a deferred tax liability of €1,379,000 equal to 29.58% (the German corporate tax rate) of the presented IPR&D). The Company classified his deferred tax liability as non-current. The book loss in Germany of €247,000 will be pro-forma considered as being equal to the taxable loss.

The net result in 2013 for uniQure Inc. (USA) translated in to taxable loss of nil as for tax purposes under Sec 195 (startup costs) all book expenses were capitalized to offset the loss. In 2014 the

# UNIQURE N.V.

## Notes to Consolidated Financial Statements (Continued)

For the Years Ended December 31, 2012, 2013 and 2014

### 26. Income Tax Expense (Continued)

net loss of €7,430,000 (\$8,937,000), based on the assumption that uniQure Inc. has started "active trade or business" where the book expenses are no longer capitalized; the pro-forma calculation of the taxable loss indicates an estimated amount of \$5.0 million (€4.2 million).

In the USA (for periods ended December 2013 and 2014) and for Germany for the period ended December 31, 2014 no tax charges or liabilities were incurred as these foreign subsidiaries were in a loss making position. No deferred tax assets have been recognized in respect of carry forward losses and the amounts presented for 2014 equal the respective Net Operating Losses available to offset future profits.

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 2005 can still be offset against profits up to and including 2014. 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 (should available tax losses carried forward be utilized) instead of the general tax rate of 25.0%. 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.

The Dutch fiscal unity has as of December 31, 2014 an estimated €145,107,000 (2013: €127,820,000) of taxable losses that can be offset in the following nine years. The expiration dates of these Dutch losses, is summarized in the following table. In the year ended December 31, 2014, the amount of unused tax losses that expired was €1,336,000 (2013: €56,000).

(€ in thousands)	2015	2016	2017	2018	2019	2020	2021	2022	2023
Loss expiring	1,838	4,228	35,608	16,709	18,127	16,476	12,398	21,099	18,624

### 27. Loss per Share

#### Basic Loss per Share

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.

	For the years ended December 31,		
	2012	2013	2014
Loss attributable to equity holders of the Company (€ in thousands)	(14,716)	(26,820)	(37,040)
Weighted average number of ordinary shares outstanding ('000)	8,637	10,796	17,121
<b>Basic loss per share (€)</b>	<b>(1.70)</b>	<b>(2.48)</b>	<b>(2.16)</b>

**UNIQURE N.V.**

## Notes to Consolidated Financial Statements (Continued)

For the Years Ended December 31, 2012, 2013 and 2014

**27. Loss per Share (Continued)****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, all potential ordinary shares had an antidilutive effect, if converted, and thus have been excluded from the computation of loss per share.

	DECEMBER 31, 2013	DECEMBER 31, 2014
Warrants	170,802	170,802
Share options under 2012 Plan	1,691,844	1,527,782
Share options under the 4D Plan	—	457,308
Share options under 2014 Plan	—	1,068,750
RSU's	—	179,068
<b>Total</b>	<b>1,862,646</b>	<b>3,403,710</b>

**28. Dividends per Share**

The Company did not declare dividends for the years ended December 31, 2014, 2013 and 2012.

**29. Commitments and Contingent Liabilities****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, the Company 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.

**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 €542,000.

# UNIQUE N.V.

## Notes to Consolidated Financial Statements (Continued)

For the Years Ended December 31, 2012, 2013 and 2014

### 29. Commitments and Contingent Liabilities (Continued)

The lease expenditure charged to the income statement for Amsterdam-based operating leases amounts to €550,000 in the year ended December 31, 2014 (2013: €542,000, 2012: €542,000). The future aggregate minimum lease payments under non-cancellable operating leases are as follows:

	DECEMBER 31, 2013	DECEMBER 31, 2014
	(€ in thousands)	
No later than 1 year	1,243	1,918
Later than 1 year and no later than 5 years	6,053	6,394
Later than 5 years	7,927	7,285
<b>Total</b>	<b>15,223</b>	<b>15,597</b>

On July 24, 2013 uniQure entered into an agreement for the lease of facilities at 113 Hartwell Avenue, Lexington, Massachusetts, United States from November 5, 2013 until November 5, 2023. uniQure has an option to extend the lease for up to an additional 10 years. The aggregate annual lease payments for the period to November 5, 2023 amount to \$18,937,000 (€ 13,756,000), including an initial rent-free period of seven months from the commencement of the lease which was effective at November 5, 2013.

The lease payments under the Lexington-based operating lease will be expensed on a straight line basis over the full duration of the lease, taking into account the Lease Incentives as received from the landlord (for a total of \$7,259,000 (€5,972,000); This results in a monthly expense of \$92,680 (€76,249); During 2014 the Company expensed a total amount of \$1,113,000 (€841,000). As of December 31, 2014 the Company recorded a total deferred rent of \$7,454,000 (€6,132,000), with a current element of \$577,000 (€475,000) and a long-term element of \$6,877,000 (€5,658,000).

### Supplier Commitments

uniQure has entered into commitments to suppliers of equipment to be installed in the Company's Lexington facility for an amount of €1.2 million as per December 31, 2014.

### 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 commitments are as follows:

	DECEMBER 31, 2013	DECEMBER, 31 2014
	(€ in thousands)	
No later than 1 year	327	306
Later than 1 year and no later than 5 years	—	—
Later than 5 years	—	—
<b>Total</b>	<b>327</b>	<b>306</b>



**UNIQUE N.V.**

## Notes to Consolidated Financial Statements (Continued)

For the Years Ended December 31, 2012, 2013 and 2014

**29. Commitments and Contingent Liabilities (Continued)****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, 2014 was €5,822,000 (2013: €5,508,000), 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. The Company has commenced repayments of the TOK and associated interest from the commercialization proceeds of Glybera arising from the agreement with Chiesi. During the period ended December 31, 2013 the Company recognized an amount of €800,000 (2014: € nil) as a charge in the consolidated statement of comprehensive income within cost 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, 2014, the total amount of the liability was €1,170,000, representing the amount of the original advance together with accrued interest (2013: €1,063,000). 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 December 11, 2013, the Company 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

**UNIQURE N.V.****Notes to Consolidated Financial Statements (Continued)**

For the Years Ended December 31, 2012, 2013 and 2014

**29. Commitments and Contingent Liabilities (Continued)**

consulting services provided to the Company 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. The amount claimed is \$100,000 plus 2.5% of all proceeds, including equity investments, the Company receives from Chiesi pursuant to its collaboration agreements entered into in the second quarter of 2013. The Company's engagement letter with Extera Partners contains a cap limiting the maximum liability to €5,000,000.

On May 12, 2014, the ICC appointed and confirmed a sole arbitrator. On October 1, 2014, Extera Partners LLC filed its Statement of Case which includes an estimated claim based on the formula mentioned above and on Extera's estimate of potential future revenues. A final merits hearing has been scheduled for July 2015. The Company has denied the claim and intends to vigorously defend against it.

**30. Related-Party Transactions**

In the period ended December 31, 2014 and 2013, the Management Board and other Senior Management 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.

The Company recognizes shareholders holding more than 5% of the Company's ordinary shares also as related parties. The Company's significant shareholders as at December 31, 2014 were:

Chiesi Farmaceutici S.p.A  
Coller Capital  
Fidelity Management & Research Company  
Forbion Capital Partners  
Gilde Healthcare Partners  
Lupus Alpha PE Champions

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, and resigned on November 8, 2013, is a partner of Gilde Healthcare Partners. Based on the information above, Gilde Healthcare is a related party of uniQure.

Funds affiliated with Lupus Alpha also have a material interest in the Company. Chiesi became a related party following 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 periods ended December 31, 2014, and December 31, 2013.

# UNIQUE N.V.

## Notes to Consolidated Financial Statements (Continued)

For the Years Ended December 31, 2012, 2013 and 2014

### 30. Related-Party Transactions (Continued)

The 2012 convertible loan from Forbion, Gilde, Lupus Alpha, Grupo Netco and affiliates, and Collier Capital, as amended in March 2013, generated in the period ended December 31, 2013 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. In the period ended December 31, 2014, the Company recorded €29,000 as board related expenses that were reimbursed to Forbion (2013: €11,000).

In the period ended December 31, 2014, the Company received funds from Chiesi for issued invoices totaling €3,292,000 (2013: €1,222,000).

As of December 31, 2014, the Company had a receivable outstanding with Chiesi for €2,404,000.

### 31. Key Management Compensation

The aggregate remuneration of the Supervisory Directors amounted to €340,000 in 2014 (2013: €400,000, 2012: €255,000) as follows:

YEAR ENDED DECEMBER 31, 2014 (in thousands €)	SALARY	BONUS	SHARE-BASED PAYMENTS(1)	PENSIONS	ADVISOR'S FEE	2014 TOTAL	2013 TOTAL	2012 TOTAL
Ferdinand Verdonck	—	—	54	—	53	107	281	43
Sander van Deventer(2)	—	—	9	—	13	22	—	8
Joseph Feczko	—	—	17	—	32	49	58	69
Edwin de Graaf(3)	—	—	—	—	—	—	—	—
Francois Meyer(4)	—	—	17	—	34	51	58	69
Sander Slootweg(3)	—	—	9	—	6	15	—	—
Philippe Van Holle(5)	—	—	—	—	—	—	(40)	66
Paula Soteropoulos(6)	—	—	38	—	40	78	43	—
Robert Coffin(7)	—	—	—	—	—	—	—	—
Will Lewis(8)	—	—	18	—	—	18	—	—
<b>Total</b>	<b>—</b>	<b>—</b>	<b>162</b>	<b>—</b>	<b>178</b>	<b>340</b>	<b>400</b>	<b>255</b>

- (1) The share-based payment reflects the value of equity- settled share options expensed 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 until after the IPO.
- (3) Appointed April 5, 2012. Mr de Graaf resigned on November 8, 2013; Mr Slootweg received no remuneration until after the IPO
- (4) Resigned December 31, 2014
- (5) Resigned January 1, 2013
- (6) Appointed June 5, 2013
- (7) Appointed November 18, 2013 and resigned December 10, 2013
- (8) Appointed June 11, 2014

# UNIQUE N.V.

## Notes to Consolidated Financial Statements (Continued)

For the Years Ended December 31, 2012, 2013 and 2014

### 31. Key Management Compensation (Continued)

The table below sets out a breakdown in the remuneration for the year ended December 31, 2014 of the members of the Management Board and Senior Management:

YEAR ENDED DECEMBER 31, 2014 (in thousands €)	SHORT TERM EMPLOYEE BENEFITS	SHARE-BASED PAYMENTS(2)	POST- EMPLOYMENT BENEFITS	OTHER LONG TERM BENEFITS	TERMINATION BENEFITS	TOTAL
			(€ in thousands)			
Jörn Aldag	484	629	25	—	—	1,138
Piers Morgan(1)	162	31	—	—	—	193
Total for Management Directors	646	660	25	—	—	1,331
Senior Management	1,791	1,437	208	—	—	3,436
Total	2,437	2,097	233	—	—	4,767

(1) Piers Morgan resigned from the Company effective May 20, 2014

(2) The share-based payment reflects the value of equity- settled share options expensed during the year, as required by IFRS 2 as well as the RSU granted in 2014 to Jörn Aldag.

The total remuneration (excluding share-based payments) paid to or for the benefit of members of the Management Board and Senior Management in 2014 amounted to approximately €2,670,000 (2013: €2,017,000).

The table below sets out a breakdown in the remuneration for the year ended December 31, 2013 of the members of the Management Board and Senior Management:

YEAR ENDED DECEMBER 31, 2013 (in thousands €)	SHORT TERM EMPLOYEE BENEFITS	SHARE-BASED PAYMENTS(1)	POST- EMPLOYMENT BENEFITS	OTHER LONG TERM BENEFITS	TERMINATION BENEFITS	TOTAL
			(€ in thousands)			
Jörn Aldag	480	266	41	—	—	787
Piers Morgan	267	111	19	—	—	397
Total for Management Directors	747	377	60	—	—	1,184
Senior Management	1,101	873	109	—	—	2,083
Total	1,884	1,250	169	—	—	3,267

(1) The share-based payment reflects the value of equity- settled share options expensed during the year, as required by IFRS 2

# UNIQUE N.V.

## Notes to Consolidated Financial Statements (Continued)

For the Years Ended December 31, 2012, 2013 and 2014

### 31. Key Management Compensation (Continued)

The table below sets out a breakdown in the remuneration for the year ended December 31, 2012 of the members of the Management Board and Senior Management:

YEAR ENDED DECEMBER 31, 2012 (in thousands €)	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

### Shares and Share Options Held by Key Management

#### Options

	NUMBER OF OPTIONS AT JANUARY 1, 2014	OPTIONS GRANTED DURING THE YEAR	OPTIONS LAPSED/EXPIRED DURING THE YEAR	NUMBER OF OPTIONS AT DECEMBER 31, 2014
Jörn Aldag	337,565	112,500	—	450,065
Piers Morgan	140,652	—	—	140,652
Senior Management	674,608	220,000	—	894,608
Total	1,152,825	332,500	—	1,485,325

Piers Morgan resigned from the Company effective May 20, 2014.

#### Ordinary Shares

	NUMBER OF SHARES
Jörn Aldag	39,389
Piers Morgan	27,805
Senior Management	16,254
Total	83,448

# UNIQUE N.V.

## Notes to Consolidated Financial Statements (Continued)

For the Years Ended December 31, 2012, 2013 and 2014

### 31. Key Management Compensation (Continued)

#### Restricted Stock Units (RSU's)

	NUMBER OF RSU's AT JANUARY 1, 2014	RSU's GRANTED DURING THE YEAR	RSU's LAPSED/EXPIRED DURING THE YEAR	NUMBER OF RSU's AT DECEMBER 31, 2014
Jörn Aldag	—	179,068	—	179,068
Total	—	179,068	—	179,068

Pursuant to an agreement dated October 8, 2014 Jörn Aldag was granted, effective August 26, 2014 a total of 179,068 Restricted Stock Units. All of these RSU's will vest on the February 6, 2016.

#### Receivables and Payables Key Management

	December 31,	
(in thousands €)	2013	2014
Receivables from Senior Management	22	22
Total	22	22

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 Company's ordinary shares or on the respective employee ceasing to be employed by the Company.

### 32. Litigation and Arbitration

On December 11, 2013, the Company 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 the Company 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. The amount claimed is \$100,000 plus 2.5% of all proceeds, including equity investments, the Company received from Chiesi pursuant to its collaboration agreements entered into in the second quarter of 2013. The Company's engagement letter with Extera Partners contains a cap limiting the maximum liability to €5,000,000.

On May 12, 2014, the ICC appointed and confirmed a sole arbitrator. On October 1, 2014, Extera Partners LLC filed its Statement of Case which includes an estimated claim based on the formula mentioned above and on Extera's estimate of potential future revenues. A final merits hearing has been scheduled for July 2015. The Company has denied the claim and intends to vigorously defend against it.

### 33. Events After the Balance Sheet Date

The Company announced that Matthew Kapusta has joined uniQure's management team as Chief Financial Officer effective January 1, 2015.

**UNIQUE N.V.**

## Notes to Consolidated Financial Statements (Continued)

For the Years Ended December 31, 2012, 2013 and 2014

**33. Events After the Balance Sheet Date (Continued)**

On January 14, 2015 Treeway announced a License and Collaboration Agreement between the Company and Treeway to Develop a Gene Therapy for Amyotrophic Lateral Sclerosis (ALS). Treeway is a biotechnology company and has been founded by entrepreneurs Bernard Muller and Robbert Jan Stuit, both diagnosed with ALS. Treeway's strategy is founded on a cohesive combination of approaches that together should provide the highest likelihood of bringing successful treatments for ALS to the patient in the short term. Under the terms of the agreement there will be no upfront or milestone payments. Treeway is responsible for the development of the therapy and the Company would be entitled to receive payments for manufacturing as well as commercial rights in North and South America and Japan.

On January 21, 2015 the Company filed its Statement of Defense with the International Chamber of Commerce, in the pending litigation regarding the Extera claim.

On January 31, 2015 we entered into a collaboration and license agreement with Synpromics Ltd. for the discovery and selection of promoters with improved activity. Under this agreement, uniQure has the exclusive rights to five selected promoter sequences for driving gene expression in liver cells using AAV mediated gene therapy. Synpromics has generated a patent protected technology to create a rationally designed library of DNA fragments which can be used to assemble synthetic promoters with improved activity. Under the agreement Synpromics and uniQure collaborate in the selection of the promoters using Synpromics' protected technology to create rationally designed libraries of DNA fragments, which can be used to assemble synthetic promoters with superior characteristics. We are required to make payments for pre-clinical, clinical and regulatory milestones under this collaboration as well as low single digit royalties.

On April 6, 2015, the Company entered into agreements with BMS, which provide BMS exclusive access to uniQure's gene therapy technology platform for multiple targets in cardiovascular and other target-specific disease areas. The collaboration includes the Company's proprietary congestive heart failure gene therapy program, which has demonstrated in advanced preclinical models that it can restore the ability to synthesize S100A1, a calcium sensor and master regulator of heart function, and increase survival rates after myocardial infarction. In addition, the Company will collaborate with BMS on up to nine additional gene therapy targets addressing a broad range of cardiovascular and other target-specific disease areas. uniQure will be responsible for discovery, preclinical development, and CMC, and will provide BMS its vector technologies and access to its industrial, proprietary insect-cell based manufacturing platform. uniQure will be responsible for CMC portions of regulatory filings, and will co-operate with BMS in the preparation of all regulatory materials and interactions with regulatory authorities. BMS will be responsible for clinical development and all commercial activities across all programs.

The financial terms consist of guaranteed, near-term payments to uniQure of at least \$97 million, including an upfront payment of \$50 million to be made at the closing of the transaction. The closing of the transaction is expected to occur in the second quarter of 2015, subject to Hart-Scott-Rodino clearance and customary closing conditions, which are considered by Management to be reasonably certain of being satisfied. An additional \$15 million payment is to be received following the selection of three additional collaboration targets, in addition to the S100A1 program, within three months of the closing. In addition, an initial equity investment in uniQure will be made for a number of shares that will equal 4.9% of the total number of shares outstanding following such issuance, at a purchase price of \$33.84 per share, or at least \$32 million in total. This investment is expected to be completed in the second quarter of 2015. BMS is also obligated to make an additional equity investment in uniQure for

**UNIQUE N.V.**

Notes to Consolidated Financial Statements (Continued)

For the Years Ended December 31, 2012, 2013 and 2014

**33. Events After the Balance Sheet Date (Continued)**

a number of shares that will equal 5.0% of the total number of shares outstanding following such issuance by December 31, 2015 and will be granted two warrants to acquire up to an additional 10% equity interest, at a premium to market, based on additional targets being introduced into the collaboration. The parties have also agreed to enter into a supply contract, under which uniQure will undertake the manufacturing of all gene therapy products under the collaboration.

No other events occurred after the balance sheet date that would have a material impact on the result or financial position of uniQure.





## UNIQUE

## AMENDED AND RESTATED LOAN AND SECURITY AGREEMENT

THIS AMENDED AND RESTATED LOAN AND SECURITY AGREEMENT is made and dated as of June 26, 2014 and is entered into by and among (i) UNIQUE BIOPHARMA B.V., a private limited liability company incorporated and existing under the laws of the Netherlands, having its corporate seat at Amsterdam, the Netherlands and registered at the trade register of the Chamber of Commerce for Amsterdam under number 34275365 (“**uniQure**”), (ii) UNIQUE IP B.V., a private limited liability company incorporated and existing under the laws of the Netherlands, having its corporate seat at Amsterdam, the Netherlands and registered at the trade register of the Chamber of Commerce for Amsterdam under number 34275369 (“**uniQure IP**”), (iii) each of the subsidiaries of uniQure identified on the Schedule 1 hereto and the signature pages hereof (uniQure, uniQure IP and such subsidiaries are hereinafter collectively referred to as “**Borrower**”), (iv) UNIQUE N.V. (formerly uniQure B.V.), a public limited company incorporated and existing under the laws of the Netherlands, having its corporate seat at Amsterdam, the Netherlands and registered at the trade register of the Chamber of Commerce for Amsterdam under number 54385229, solely a party hereto for purposes of Section 3.1(a) (“**uniQure Holdings**”) and (v) HERCULES TECHNOLOGY GROWTH CAPITAL, INC., a Maryland corporation (“**Lender**”).

## RECITALS

A. **WHEREAS**, Borrower, uniQure Holdings, and Lender are party to that certain Loan and Security Agreement dated as of June 13, 2013 (as the same may have been amended, modified, supplemented or restated and in effect from time to time, the “**Existing Loan and Security Agreement**”);

B. **WHEREAS**, immediately prior to the effectiveness of this Amended and Restated Loan and Security Agreement, there is a Term Loan outstanding under the Existing Loan and Security Agreement in the aggregate principal amount of \$10,000,000 (the “**Existing Term Loan**”);

C. **WHEREAS**, Borrower desires to obtain financing to increase the aggregate amount of Term Loans to \$20,000,000 (inclusive of the Existing Term Loan) for general corporate purposes permitted pursuant to the terms of this Amended and Restated Loan and Security Agreement;

D. **WHEREAS**, the parties hereto desire to amend and restate the Existing Loan and Security Agreement upon the terms and subject to the conditions set forth herein.

**NOW, THEREFORE**, in consideration of the mutual conditions and agreements set forth in this Amended and Restated Loan and Security Agreement, and for good and valuable consideration, the receipt of which is hereby acknowledged, the parties hereto hereby agree that the Existing Loan and Security Agreement shall be amended and restated in its entirety to read as follows (it being agreed that this Amended and Restated Loan and Security Agreement shall not

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be deemed to evidence or result in a novation or repayment and reborrowing of the Existing Term Loan under the Existing Loan and Security Agreement):

NOW, THEREFORE, Borrower and Lender agree as follows:

## SECTION 1. DEFINITIONS AND RULES OF CONSTRUCTION

1.1 Unless otherwise defined herein, the following capitalized terms shall have the following meanings:

“**Account Control Agreement(s)**” means any agreement entered into by and among the Lender, US Borrower and a third party bank or other institution (including a Securities Intermediary) in which US Borrower maintains a Deposit Account or an account holding Investment Property and which grants Lender a perfected first priority security interest in the subject account or accounts.

“**ACH Authorization**” means the ACH Debit Authorization Agreement in substantially the form of Exhibit H.

“**Additional End of Term Charge**” has the meaning given to it in Section 2.6.

“**Advance**” means a Term Loan Advance.

“**Advance Date**” means the funding date of an Advance.

“**Advance Request**” means a request for an Advance submitted by Borrower to Lender in substantially the form of Exhibit A.

“**Agreement**” means this Amended and Restated Loan and Security Agreement, as amended from time to time.

“**Amortization Date**” means January 1, 2016; provided that in Lender’s sole discretion, such date may be extended to April 1, 2016.

“**Assignee**” has the meaning given to it in Section 11.12.

“**Borrower Products**” means all products, software, service offerings, technical data or technology currently being designed, manufactured or sold by Borrower or which Borrower intends to sell, license, or distribute in the future including any products or service offerings under development, collectively, together with all products, software, service offerings, technical data or technology that have been sold, licensed or distributed by Borrower since its incorporation.

“**Business Day**” is any day that is not a Saturday, Sunday or a day on which Lender is closed.

“**Cash**” means all cash and liquid funds.

“**Change in Control**” means any (i) reorganization, recapitalization, consolidation or merger (or similar transaction or series of related transactions) of uniQure Holdings, Borrower or any Subsidiary, sale or exchange of outstanding shares (or similar transaction or series of related transactions) of uniQure Holdings, Borrower or any Subsidiary in which the holders of uniQure Holdings’ Borrower’s or Subsidiary’s outstanding shares immediately before consummation of such transaction or series of related transactions do not, immediately after consummation of such transaction or series of related transactions, retain shares representing more than fifty percent (50%) of the voting power of the surviving entity of such transaction or series of related transactions (or the parent of such surviving entity if such surviving entity is wholly owned by such parent), in each case without regard to whether uniQure Holdings, Borrower or Subsidiary is the surviving entity, or (ii) sale or issuance by uniQure Holdings, Borrower of new shares of Preferred Stock of uniQure Holdings or Borrower to investors, none of whom are current investors in uniQure Holdings or Borrower, and such new shares of Preferred Stock are senior to all existing Preferred Stock and common stock of uniQure Holdings or Borrower with respect to liquidation preferences, and the aggregate liquidation preference of the new shares of Preferred Stock is more than fifty percent (50%) of the aggregate liquidation preference of all shares of Preferred Stock of uniQure Holdings or Borrower; provided, however, an Initial Public Offering shall not constitute a Change in Control.

“**Closing Date**” means June 13, 2013.

“**Collateral**” means the property described in Section 3.

“**Collateral Documents**” means the security documents described in Section 3.

“**Confidential Information**” has the meaning given to it in Section 11.11.

“**Contingent Obligation**” means, as applied to any Person, any direct or indirect liability, contingent or otherwise, of that Person with respect to (i) any indebtedness, lease, dividend, letter of credit or other obligation of another, including any such obligation directly or indirectly guaranteed, endorsed, co-made or discounted or sold with recourse by that Person, or in respect of which that Person is otherwise directly or indirectly liable; (ii) any obligations with respect to undrawn letters of credit, corporate credit cards or merchant services issued for the account of that Person; and (iii) all obligations arising under any interest rate, currency or commodity swap agreement, interest rate cap agreement, interest rate collar agreement, or other agreement or arrangement designated to protect a Person against fluctuation in interest rates, currency exchange rates or commodity prices; provided, however, that the term “Contingent Obligation” shall not include endorsements for collection or deposit in the ordinary course of business. The amount of any Contingent Obligation shall be deemed to be an amount equal to the stated or determined amount of the primary obligation in respect of which such Contingent Obligation is made or, if not stated or determinable, the maximum reasonably anticipated liability in respect thereof as determined by such Person in good faith; provided, however, that such amount shall not in any event exceed the maximum amount of the obligations under the guarantee or other support arrangement.

“**Copyright License**” means any written agreement granting any right to use any Copyright or Copyright registration, now owneded or hereafter acquired by Borrower or in which Borrower now holds or hereafter acquires any interest.

“**Copyrights**” means all copyrights, whether registered or unregistered, held by the Borrower pursuant to the laws of the Netherlands, or of any other country.

“**Deposit Accounts**” means any “deposit accounts,” including any checking account, savings account, or certificate of deposit and any deposit account as defined in the UCC.

“**End of Term Charge**” is defined in Section 2.5.

“**Event of Default**” has the meaning given to it in Section 9.

“**Existing Loan and Security Agreement**” as defined in Recital A.

“**Existing Term Loan**” as defined in Recital B.

“**Facility Charge**” means one and one-quarter of one percent (1.25%) of the original principal amount of the Term Loan advanced on the Closing Date.

“**Financial Statements**” has the meaning given to it in Section 7.1.

“**Funding Documents**” means the following: (i) a certificate of good standing for US Borrower from its state of incorporation and from all other US jurisdictions in which it does business, and, if applicable under the laws of any non-US jurisdiction, a certificate of good standing or the equivalent for Borrower and US Borrower from all non-US jurisdictions in which such entity does business, in each case where the failure to be qualified to do business would have a Material Adverse Effect; (ii) completed Schedules and Exhibits to this Agreement; (iii) executed originals of the following: (x) the Account Control Agreements, and any other documents executed in connection with the Secured Obligations or the transactions contemplated hereby, as the same may from time to time be amended, modified, supplemented or restated and (y) the Perfection Certificate; (iv) legal opinion of Lender’s counsel; (v) the insurance policies and/or endorsements required pursuant to Section 6.1 hereof; (vi) documents, releases, terminations, and other instruments as may be necessary or proper to release any creditor’s Lien in the Intellectual Property of Borrower including, without limitation, UCC financing statement amendments and appropriate filings with any appropriate register or authority in any jurisdiction; and (vii) and all other documents and instruments reasonably required by Lender to effectuate the transactions contemplated hereby or to create and perfect the Liens of Lender with respect to all Collateral, in all cases in form and substance reasonably acceptable to Lender.

“**IFRS**” are the International Financial Reporting Standards, a collection of guidelines and rules set by the International Accounting Standards Board ([www.iasb.org](http://www.iasb.org)) which are applicable to the circumstances as of the date of determination.

“**Indebtedness**” means indebtedness of any kind, including (a) all indebtedness for borrowed money or the deferred purchase price of property or services (excluding trade credit entered into in the ordinary course of business due within sixty (60) days), including

reimbursement and other obligations with respect to surety bonds and letters of credit, (b) all obligations evidenced by notes, bonds, debentures or similar instruments, (c) all capital lease obligations, and (d) all Contingent Obligations.

“**Initial Public Offering**” means the initial firm commitment underwritten offering of uniQure Holdings common stock pursuant to a registration statement under the Securities Act of 1933 filed with and declared effective by the Securities and Exchange Commission.

“**Insolvency Proceeding**” is any proceeding by or against any Person under the Dutch Bankruptcy Act, or any other bankruptcy or insolvency law, including assignments for the benefit of creditors, compositions, extensions generally with its creditors, or proceedings seeking reorganization, arrangement, or other relief.

“**Intellectual Property**” means any and all intellectual property rights in any country or jurisdiction, including but not limited to all of Borrower’s Copyrights; Trademarks; Patents; Licenses; trade secrets and inventions; mask works, utility models, layout-designs (topographies) of integrated circuits, know-how, industrial designs, neighbouring rights, database rights or other rights in compilations of data, trade names, internet domain names, plant variety rights and any and all rights of a similar nature, either (i) now known, contemplated or unforeseen, (ii) having a statutory basis or existing under equity, common law or otherwise, (iii) registered, deposited, filed or not, and including any and all rights in connection with applications for or rights to apply for or acquire any and all of such rights.

“**Investment**” means any beneficial ownership (including stock, partnership or limited liability company interests) of or in any Person, or any loan, advance or capital contribution to any Person or the acquisition of all, or substantially all, of the assets of another Person.

“**Joinder Agreements**” means for each Subsidiary, a completed and executed Joinder Agreement in substantially the form attached hereto as Exhibit G.

“**Lender**” has the meaning given to it in the preamble to this Agreement.

“**License**” means any Copyright License, Patent License, Trademark License or other license of rights or interests.

“**Lien**” means any mortgage, deed of trust, pledge, hypothecation, assignment for security, security interest, encumbrance, levy, lien or charge of any kind, whether voluntarily incurred or arising by operation of law or otherwise, against any property, any conditional sale or other title retention agreement, and any lease in the nature of a security interest.

“**Loan**” means the Advance made under this Agreement.

“**Loan Documents**” means this Agreement, the Notes (if any), the ACH Authorization, the Account Control Agreements, the Joinder Agreements, all UCC Financing Statements, the Warrant Agreement, any intellectual property security agreement, and any other documents executed in connection with the Secured Obligations or the transactions contemplated hereby, as the same may from time to time be amended, modified, supplemented or restated.

“**Material Adverse Effect**” means a material adverse effect upon: (i) the business, operations, properties, assets or condition (financial or otherwise) of Borrower and its Subsidiaries, taken as a whole, other than in and of itself (x) the expenditure of cash in the ordinary course, or (y) adverse results of a clinical trial or program or the denial, delay or limitation of approval of, or taking of any other regulatory action by, the United States Food and Drug Administration or any other governmental entity with respect to any biologic product or drug; or (ii) the ability of Borrower to perform the Secured Obligations when due in accordance with the terms of the Loan Documents, or the ability of Lender to enforce any of its rights or remedies with respect to the Secured Obligations; or (iii) the Collateral or Lender’s Liens on the Collateral or the priority of such Liens

“**Maximum Rate**” shall have the meaning assigned to such term in Section 2.2.

“**Note(s)**” means a promissory note or promissory notes to evidence Lender’s Loans.

“**Patent License**” means any written agreement granting any right with respect to any invention on which a Patent is in existence or a Patent application is pending, in which agreement Borrower now holds or hereafter acquires any interest.

“**Patents**” means any patent in the Netherlands or in any other country, all registrations and recordings thereof, and all applications for patents of, or rights corresponding thereto, in the Netherlands or any other country.

“**Permitted Indebtedness**” means: (i) Indebtedness of Borrower in favor of Lender arising under this Agreement or any other Loan Document; (ii) Indebtedness existing on the Closing Date which is disclosed in Schedule 1A; (iii) Indebtedness of up to \$250,000 outstanding at any time secured by a Lien described in clause (vii) of the defined term “Permitted Liens,” provided such Indebtedness does not exceed the lesser of the cost or fair market value of the equipment financed with such Indebtedness; (iv) Indebtedness to trade creditors incurred in the ordinary course of business, including Indebtedness incurred in the ordinary course of business with corporate credit cards; (v) Indebtedness that also constitutes a Permitted Investment; (vi) Subordinated Indebtedness; (vii) reimbursement obligations in connection with letters of credit that are secured by cash or cash equivalents and issued on behalf of the Borrower or a Subsidiary thereof in an amount not to exceed \$200,000 at any time outstanding, (viii) other Indebtedness in an amount not to exceed \$100,000

at any time outstanding, and (ix) extensions, refinancings and renewals of any items of Permitted Indebtedness, provided that the principal amount is not increased or the terms modified to impose materially more burdensome terms upon Borrower or its Subsidiary, as the case may be.

**“Permitted Investment”** means: (i) Investments existing on the Closing Date which are disclosed in Schedule 1B; (ii) (a) marketable direct obligations issued or unconditionally guaranteed by any agency or any country thereof maturing within one year from the date of acquisition thereof, (b) commercial paper maturing no more than one year from the date of creation thereof and currently having a rating of at least A-2 or P-2 from either Standard & Poor’s Corporation or Moody’s Investors Service, (c) certificates of deposit issued by any bank with assets of at least \$500,000,000 maturing no more than one year from the date of investment therein, and (d) money market accounts; (iii) repurchases of stock from former employees,

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directors, or consultants of Borrower under the terms of applicable repurchase agreements at the original issuance price of such securities in an aggregate amount not to exceed \$250,000 in any fiscal year, provided that no Event of Default has occurred, is continuing or would exist after giving effect to the repurchases; (iv) Investments accepted in connection with Permitted Transfers; (v) Investments (including debt obligations) received in connection with the bankruptcy or reorganization of customers or suppliers and in settlement of delinquent obligations of, and other disputes with, customers or suppliers arising in the ordinary course of Borrower’s business; (vi) Investments consisting of notes receivable of, or prepaid royalties and other credit extensions, to customers and suppliers who are not affiliates, in the ordinary course of business, provided that this subparagraph (vi) shall not apply to Investments of Borrower in any Subsidiary; (vii) Investments consisting of loans not involving the net transfer on a substantially contemporaneous basis of cash proceeds to employees, officers or directors relating to the purchase of capital stock of Borrower pursuant to employee stock purchase plans or other similar agreements approved by Borrower’s board of directors; (viii) Investments consisting of employee travel advances, employee relocation loans and other employee loans and advances in the ordinary course of business; (ix) Investments in newly-formed Subsidiaries organized in the Netherlands or any other country, provided that such Subsidiaries enter into a Joinder Agreement promptly after their formation by Borrower and execute such other documents as shall be reasonably requested by Lender; (xi) joint ventures or strategic alliances in the ordinary course of Borrower’s business consisting of the nonexclusive licensing of technology, the development of technology or the providing of technical support, provided that any cash Investments by Borrower do not exceed \$100,000 in the aggregate in any fiscal year; and (xii) other Investments that do not exceed \$250,000 in the aggregate.

**“Permitted Liens”** means any and all of the following: (i) Liens in favor of Lender; (ii) Liens existing on the Closing Date which are disclosed in Schedule 1C; (iii) Liens for taxes, fees, assessments or other governmental charges or levies, either not delinquent or being contested in good faith by appropriate proceedings; provided, that Borrower maintains adequate reserves therefor in accordance with IFRS; (iv) Liens securing claims or demands of materialmen, artisans, mechanics, carriers, warehousemen, landlords and other like Persons arising in the ordinary course of Borrower’s business and imposed without action of such parties; provided, that the payment thereof is not yet required; (v) Liens arising from judgments, decrees or attachments in circumstances which do not constitute an Event of Default hereunder; (vi) the following deposits, to the extent made in the ordinary course of business: deposits under worker’s compensation, unemployment insurance, social security and other similar laws, or to secure the performance of bids, tenders or contracts (other than for the repayment of borrowed money) or to secure indemnity, performance or other similar bonds for the performance of bids, tenders or contracts (other than for the repayment of borrowed money) or to secure statutory obligations (other than liens arising under environmental liens) or surety or appeal bonds, or to secure indemnity, performance or other similar bonds; (vii) Liens on equipment or software or other intellectual property constituting purchase money liens and liens in connection with capital leases securing Indebtedness permitted in clause (iii) of “Permitted Indebtedness”; (viii) Liens incurred in connection with Subordinated Indebtedness; (ix) leasehold interests in leases or subleases and licenses granted in the ordinary course of business and not interfering in any material respect with the business of the licensor; (x) Liens in favor of customs and revenue authorities arising as a matter of law to secure payment of custom duties that are promptly paid on or before the date they become due; (xi) Liens on insurance proceeds securing the payment of

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financed insurance premiums that are promptly paid on or before the date they become due (provided that such Liens extend only to such insurance proceeds and not to any other property or assets); (xii) statutory and common law rights of set-off and other similar rights as to deposits of cash and securities in favor of banks, other depository institutions and brokerage firms; (xiii) easements, zoning restrictions, rights-of-way and similar encumbrances on real property imposed by law or arising in the ordinary course of business so long as they do not materially impair the value or marketability of the related property; (xiv) Liens on cash or cash equivalents securing obligations permitted under clause (vii) of the definition of Permitted Indebtedness; and (xv) Liens incurred in connection with the extension, renewal or refinancing of the indebtedness secured by Liens of the type described in clauses (i) through (xi) above; provided, that any extension, renewal or replacement Lien shall be limited to the property encumbered by the existing Lien and the principal amount of the indebtedness being extended, renewed or refinanced (as may have been reduced by any payment thereon) does not increase.

**“Permitted Transfers”** means (i) sales of inventory in the normal course of business, (ii) exclusive licenses and similar arrangements for the use of Intellectual Property in the ordinary course of business that could not result in a legal transfer of title of the licensed property (iii) dispositions of worn-out, obsolete or surplus equipment that is, in the reasonable judgment of Borrower, no longer economically practicable to maintain or useful in the ordinary course of business of Borrower, (iv) other Transfers of assets having a fair market value of not more than \$250,000 in the aggregate in any fiscal year and (v) the entering into the commercialization agreement, the co-development and license agreement and any other related documents by and among uniQure and Chiesi Farmaceutici S.p.A.

**“Person”** means any individual, sole proprietorship, partnership, joint venture, trust, unincorporated organization, association, corporation, limited liability company, institution, other entity or government.

**“Preferred Stock”** means at any given time any equity security issued by Borrower that has any rights, preferences or privileges senior to uniQure Holdings’ or Borrower’s common stock.

**“Prepayment Charge”** shall have the meaning assigned to such term in Section 2.4.

**“Prime Rate”** means the “prime rate” as reported in *The Wall Street Journal*, and if not reported, then the prime rate most recently reported in *The Wall Street Journal*.

**“Restatement Date”** shall mean June 26, 2014.

“**Second Advance End of Term Charge**” is defined in Section 2.6.

“**Second Advance Facility Charge**” means one percent (1.00%) of the original principal amount of the aggregate principal amount of the Term Loans advanced pursuant to the Loan Documents.

“**Secured Obligations**” means Borrower’s obligations under this Agreement and any Loan Document, including any obligation to pay any amount now owing or later arising.

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“**Subordinated Indebtedness**” means Indebtedness subordinated to the Secured Obligations in amounts and on terms and conditions satisfactory to Lender in its sole discretion.

“**Subsequent Financing**” means the closing of any Borrower financing which becomes effective after the Closing Date and results in aggregate proceeds to Borrower of at least Ten Million Dollars (\$10,000,000).

“**Subsidiary**” means an entity, whether corporate, partnership, limited liability company, joint venture or otherwise, in which Borrower owns or controls 50% or more of the outstanding voting securities, including each entity listed on Schedule 1 hereto.

“**Term Loan**” is each of and collectively (i) the Existing Term Loan in the original principal amount of \$10,000,000 advanced by Lender to Borrower on the Closing Date and (ii) the term loan in the original principal amount of \$10,000,000 advanced by Lender to Borrower on the Restatement Date.

“**Term Loan Advance**” means an advance of a Term Loan.

“**Term Loan Interest Rate**” means (i) for any day prior to the Restatement Date, a floating per annum rate of interest equal to the greater of either (a) eleven and eighty-five one-hundredths of one percent (11.85%), or (b) the sum of (1) eleven and eighty-five one-hundredths of one percent (11.85%), plus (2) the Prime Rate minus three and one quarter of one percent (3.25%) and (ii) for any day on or after the Restatement Date, a floating per annum rate of interest equal to the greater of either (a) ten and one quarter of one percent (10.25%), or (b) the sum of (1) ten and one quarter of one percent (10.25%), plus (2) the Prime Rate minus five and one quarter of one percent (5.25%). The Term Loan Interest Rate will change from time to time on the day the Prime Rate changes.

“**Term Loan Maturity Date**” means June 30, 2018.

“**Trademark License**” means any written agreement granting any right to use any Trademark or Trademark registration, now owned or hereafter acquired by Borrower or in which Borrower now holds or hereafter acquires any interest.

“**Trademarks**” means all trademarks (registered, common law or otherwise) and any applications in connection therewith, including registrations, recordings and applications with any appropriate register or authority in any jurisdiction.

“**UCC**” means the Uniform Commercial Code as the same is, from time to time, in effect in the State of California; provided, that in the event that, by reason of mandatory provisions of law, any or all of the attachment, perfection or priority of, or remedies with respect to, Lender’s Lien on any Collateral is governed by the Uniform Commercial Code as the same is, from time to time, in effect in a jurisdiction other than the State of California, then the term “UCC” shall mean the Uniform Commercial Code as in effect, from time to time, in such other jurisdiction solely for purposes of the provisions thereof relating to such attachment, perfection, priority or remedies and for purposes of definitions related to such provisions.

“**US Borrower**” means **uniQure, Inc.**, as identified on the Schedule 1 hereto.

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“**Warrant Agreement**” means the Warrant Agreement dated as of September 20, 2013 by and between uniQure Holdings and Lender.

Unless otherwise specified, all references in this Agreement or any Annex or Schedule hereto to a “Section,” “subsection,” “Exhibit,” “Annex,” or “Schedule” shall refer to the corresponding Section, subsection, Exhibit, Annex, or Schedule in or to this Agreement. Unless otherwise specifically provided herein, any accounting term used in this Agreement or the other Loan Documents shall have the meaning customarily given such term in accordance with IFRS, and all financial computations hereunder shall be computed in accordance with IFRS, consistently applied. Unless otherwise defined herein or in the other Loan Documents, terms that are used herein or in the other Loan Documents and defined in the UCC shall have the meanings given to them in the UCC.

## **SECTION 2.     THE LOANS**

### **2.1     Term Loans.**

(a)     Advances. Subject to the terms and conditions of this Agreement, Borrower (i) requested a Term Loan Advance in the original principal amount of \$10,000,000, which advance was funded on the Closing Date and (ii) shall request a Term Loan Advance in the principal amount of \$10,000,000 to be funded on the Restatement Date. Proceeds of an Advance shall be deposited into an account that is subject to a security interest in favor of Lender, perfected by an Account Control Agreement.

(b)     Advance Request. To obtain a Term Loan Advance, Borrower shall complete, sign and deliver to Lender an Advance Request (at least five Business Days before the Advance Date). Lender shall fund a Term Loan Advance in the manner requested by the Advance Request provided that each of the conditions precedent to such Term Loan Advance is satisfied as of the requested Advance Date.

(c)     Interest. The principal balance of each Term Loan Advance shall bear interest thereon from such Advance Date at the Term Loan Interest Rate based on a year consisting of 360 days, with interest computed daily based on the actual number of days elapsed. The Term Loan

Interest Rate will float and change on the day the Prime Rate changes from time to time.

(d) Payment. Borrower will pay interest on each Term Loan Advance on the first Business Day of each month, beginning the month after the applicable Advance Date. Commencing on the Amortization Date, and continuing on the first Business Day of each month thereafter, Borrower shall repay the aggregate principal balance of Term Loan Advances that are outstanding in 30 equal monthly installments of principal and interest (mortgage style). The entire outstanding principal balance of the Term Loan Advances and all accrued but unpaid interest hereunder, and all other Secured Obligations with respect to the Term Loan Advances, shall be due and payable on Term Loan Maturity Date. Borrower shall make all payments under this Agreement without setoff, recoupment or deduction and regardless of any counterclaim or defense. Lender will initiate debit entries to the Borrower's account as authorized on the ACH Authorization on each payment date of all periodic

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obligations payable to Lender under the Term Loan Advances. Once repaid, the Term Loan Advances or any portion thereof may not be re-borrowed.

2.2 Maximum Interest. Notwithstanding any provision in this Agreement or any other Loan Document, it is the parties' intent not to contract for, charge or receive interest at a rate that is greater than the maximum rate permissible by law that a court of competent jurisdiction shall deem applicable hereto (which under the laws of the State of California shall be deemed to be the laws relating to permissible rates of interest on commercial loans) (the "Maximum Rate"). If a court of competent jurisdiction shall finally determine that Borrower has actually paid to Lender an amount of interest in excess of the amount that would have been payable if all of the Secured Obligations had at all times borne interest at the Maximum Rate, then such excess interest actually paid by Borrower shall be applied as follows: first, to the payment of the Secured Obligations consisting of the outstanding principal of the Term Loan Advances; second, after all principal is repaid, to the payment of Lender's accrued interest, costs, expenses, professional fees and any other Secured Obligations; and third, after all Secured Obligations are repaid, the excess (if any) shall be refunded to Borrower.

2.3 Default Interest. In the event any payment is not paid on the scheduled payment date, an amount equal to five percent (5%) of the past due amount shall be payable on demand. In addition, upon the occurrence and during the continuation of an Event of Default hereunder, all Secured Obligations, including principal, interest, compounded interest, and professional fees, shall bear interest at a rate per annum equal to the rate set forth in Section 2.1(c), plus five percent (5%) per annum. In the event any interest is not paid when due hereunder, delinquent interest shall be added to principal and shall bear interest on interest, compounded at the rate set forth in Section 2.1(c).

2.4 Prepayment. At its option upon at least five (5) Business Days prior notice to Lender, Borrower may prepay all, but not less than all, of the outstanding Advance by paying the entire outstanding principal balance, all accrued and unpaid interest thereon, all unpaid Lender's fees and expenses accrued to the date of the repayment (including, without limitation, the End of Term Charge) together with a prepayment charge equal to the following percentage of the Advance amount being prepaid: if such Advance amounts are prepaid in any of the first twelve (12) months following the Restatement Date, two percent (2.00%); after twelve (12) months following the Restatement Date but prior to twenty four (24) months following the Restatement Date, one and one half percent (1.5%); and after twenty four (24) months following the Restatement Date but prior to the Term Loan Maturity Date, one percent (1%) (each, a "**Prepayment Charge**"). Borrower agrees that the Prepayment Charge is a reasonable calculation of Lender's lost profits in view of the difficulties and impracticality of determining actual damages resulting from an early repayment of the Advances. Borrower shall prepay the outstanding amount of all principal and accrued interest through the prepayment date and all unpaid Lender's fees and expenses accrued to the date of the repayment (including the End of Term Charge) together with a Prepayment Charge upon the occurrence of a Change in Control.

2.5 End of Term Charge. On the earliest to occur of (i) October 1, 2016, (ii) the date that Borrower prepays the outstanding Secured Obligations, or (iii) the date that the Secured Obligations become due and payable, Borrower shall pay Lender a charge equal to \$345,000 (the

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"**End of Term Charge**"). Notwithstanding the required payment date of such charge, it shall be deemed earned by Lender as of the Closing Date.

2.6 Additional End of Term Charge. On the earliest to occur of (i) the Term Loan Maturity Date, (ii) the date that Borrower prepays the outstanding Secured Obligations, or (iii) the date that the Secured Obligations become due and payable, Borrower shall pay Lender a additional charge equal to \$250,000 (the "**Second Advance End of Term Charge**"). Notwithstanding the required payment date of such charge, it shall be deemed earned by Lender as of the Restatement Date.

2.7 Notes. If so requested by Lender by written notice to Borrower, then Borrower shall execute and deliver to Lender (and/or, if applicable and if so specified in such notice, to any person who is an assignee of Lender pursuant to Section 11.12) (promptly after the Borrower's receipt of such notice) a Note or Notes to evidence Lender's Loans.

2.8 Commitment Fee; Facility Charge. The parties acknowledge and agree that Borrower paid to Lender (i) a commitment fee of \$45,000 on or before the Closing Date, and that such commitment fee was fully earned on the Closing Date and non-refundable regardless of the early termination of this Agreement and (ii) the Facility Charge of \$125,000 on the Closing Date, and that such Facility Charge was fully earned on the Closing Date and non-refundable regardless of the early termination of this Agreement.

### **SECTION 3. SECURITY INTEREST**

3.1 As security for the prompt, complete and indefeasible payment when due (whether on the payment dates or otherwise) of all the Secured Obligations:

(a) uniQure Holdings grants to Lender a first ranking right of pledge on its shares in uniQure and uniQure IP;

(b) uniQure grants to Lender a first ranking right of pledge on its shares in its Dutch subsidiaries identified on the Schedule 1 hereto and a security interest in 100% of the capital stock of US Borrower;

(c) Borrower (excluding US Borrower) grants to Lender a first ranking right of pledge on its (a) trade, intercompany and insurance receivables; (b) movable assets and (c) Deposit Accounts; and

(d) US Borrower grants to Lender a security interest in all of US Borrower's right, title, and interest in and to the following personal property whether now owned or hereafter acquired: (a) receivables; (b) equipment; (c) fixtures; (d) general intangibles (except as described below); (e) inventory; (f) Investment property; (g) Deposit Accounts; (h) Cash; (i) Goods; and all other tangible and intangible personal property of US Borrower whether now or hereafter owned or existing, leased, consigned by or to, or acquired by, US Borrower and wherever located, and any of Borrower's property in the possession or under the control of Lender; and, to the extent not otherwise included, all Proceeds of each of the foregoing and all accessions to, substitutions and replacements for, and rents, profits and products of each of the foregoing (collectively, the "Collateral").

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3.2 Notwithstanding anything in this Agreement or any other Loan Document to the contrary, in no event shall the Collateral include, and the Borrower shall not be deemed to have granted a security interest in: (i) Intellectual Property; provided, however, that the Collateral shall include all accounts and general intangibles that consist of rights to payment and proceeds from the sale, licensing or disposition of all or any part, or rights in, the Intellectual Property (the "Rights to Payment"); or (ii) any of the Borrower's rights or interests in or under, any license, contract, permit, instrument, security or franchise to which the Borrower is a party or any of its rights or interests thereunder to the extent, but only to the extent, that such a grant would, under the terms of such license, contract, permit, instrument, security or franchise, result in a breach of the terms of, or constitute a default under, such license, contract, permit, instrument, security or franchise (other than to the extent that any such term would be rendered ineffective pursuant to the UCC or any other applicable law (including the Dutch and the United States Bankruptcy Code) or principles of equity); provided, that immediately upon the ineffectiveness, lapse or termination of any such provision the Collateral shall include, and the Borrower shall be deemed to have granted a security interest in, all the rights and interests described in the foregoing clause (ii) as if such provision had never been in effect. Notwithstanding the foregoing, if a judicial authority (including a U.S. Bankruptcy Court) holds that a security interest in the underlying Intellectual Property is necessary to have a security interest in the Rights to Payment, then the Collateral shall automatically, and effective as of the date of this Agreement, include the Intellectual Property to the extent necessary to permit perfection of Lender's security interest in the Rights to Payment.

#### **SECTION 4. CONDITIONS PRECEDENT TO LOANS**

The obligation of Lender to make the Term Loan Advances hereunder is subject to the satisfaction by Borrower of the following conditions, which conditions were satisfied or waived by Lender on or prior to the applicable Advance Date:

4.1 Closing Documents. On or prior to the Advance Date, Borrower shall have delivered to Lender the following:

- (a) executed originals of the Loan Documents, the Collateral Documents and the ACH Authorization;
- (b) copies of resolutions of Borrower's board of directors and general meeting of shareholders evidencing approval of (i) the Loan and other transactions evidenced by the Loan Documents;
- (c) copies of the current articles of association of Borrower;
- (d) Second Advance Facility Charge on the Restatement Date and reimbursement of Lender's current expenses reimbursable pursuant to this Agreement; and
- (e) receipt of the Funding Documents and satisfaction of all conditions precedent thereto;

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(f) Lender shall have received (i) an Advance Request for the relevant Advance as required by 2.2(b), duly executed by Borrower's Chief Executive Officer or Chief Financial Officer, and (ii) any other documents Lender may reasonably request.

(g) The representations and warranties set forth in this Agreement and in Section 5 shall be true and correct in all material respects on and as of the Advance Date with the same effect as though made on and as of such date, except to the extent such representations and warranties expressly relate to an earlier date.

(h) Borrower shall be in compliance with all the terms and provisions set forth herein and in each other Loan Document on its part to be observed or performed, and at the time of and immediately after such Advance no Event of Default shall have occurred and be continuing.

(i) The Advance Request shall be deemed to constitute a representation and warranty by Borrower on the Advance Date as to the matters specified in Section 4.2 and as to the matters set forth in the Advance Request.

The parties acknowledge and agree that the Collateral Documents were executed and delivered by Borrower to Lender on the Closing Date.

4.2 No Default. As of the Advance Date, (i) no fact or condition exists that would (or would, with the passage of time, the giving of notice, or both) constitute an Event of Default and (ii) no event that has had or could reasonably be expected to have a Material Adverse Effect has occurred and is continuing.

#### **SECTION 5. REPRESENTATIONS AND WARRANTIES OF BORROWER**

Borrower represents and warrants that:



5.1 Corporate Status. Borrower is a private limited liability company duly incorporated and existing under the laws of the Netherlands, and is duly qualified as a foreign corporation in all jurisdictions in which the nature of its business or location of its properties require such qualifications and where the failure to be qualified could reasonably be expected to have a Material Adverse Effect. Borrower's present name, former names (if any), locations, place of formation, tax identification number, organizational identification number and other information are correctly set forth in Exhibit C, as may be updated by Borrower in a written notice (including any Compliance Certificate) provided to Lender after the Closing Date. US Borrower is corporation duly organized, legally existing and in good standing under the laws of the State of Delaware, and is duly qualified as a foreign corporation in all jurisdictions in which the nature of its business or location of its properties require such qualifications and where the failure to be qualified could reasonably be expected to have a Material Adverse Effect.

5.2 Collateral. Borrower owns the Collateral (other than that described in Section 3.1(a)) and the Intellectual Property, free of all Liens, except for Permitted Liens. Borrower has the power and authority to grant to Lender a Lien in the Collateral (other than that described in Section 3.1(a)) as security for the Secured Obligations.

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5.3 Consents. Borrower's execution, delivery and performance of the Notes (if any), this Agreement and all other Loan Documents, (i) have been duly authorized by all necessary corporate action of Borrower, (ii) will not result in the creation or imposition of any Lien upon the Collateral, other than Permitted Liens and the Liens created by this Agreement and the other Loan Documents, (iii) do not violate any provisions of Borrower's articles of association, or any, law, regulation, order, injunction, judgment, decree or writ to which Borrower is subject and (iv) except as described on Schedule 5.3, do not violate any contract or agreement or require the consent or approval of any other Person which has not already been obtained. The individual or individuals executing the Loan Documents are duly authorized to do so.

5.4 Material Adverse Effect. No event that has had or could reasonably be expected to have a Material Adverse Effect has occurred and is continuing. Borrower is not aware of any event likely to occur that is reasonably expected to result in a Material Adverse Effect.

5.5 Actions Before Governmental Authorities. Except as described on Schedule 5.5, there are no actions, suits or proceedings at law or in equity or by or before any governmental authority now pending or, to the knowledge of Borrower, threatened against or affecting Borrower or its property (i) which seek to prevent, enjoin, hinder or delay the transactions contemplated by the Loan Documents or (ii) as to which there is a reasonable possibility of an adverse determination and which, if adversely determined, would reasonably be expected to, individually or in the aggregate, have a material adverse effect on Borrower's business.

5.6 Laws. Borrower, to its knowledge, is not in violation of any law, rule or regulation, or in default with respect to any judgment, writ, injunction or decree of any governmental authority, where such violation or default is reasonably expected to result in a Material Adverse Effect. Borrower, to its knowledge, is not in default in any manner under any provision of any agreement or instrument evidencing indebtedness, or any other material agreement to which it is a party or by which it is bound and for which such default would reasonably be expected to have a material adverse effect on Borrower's business.

5.7 Information Correct and Current. No information, report, Advance Request, financial statement, exhibit or schedule furnished, by or on behalf of Borrower to Lender in connection with any Loan Document or included therein or delivered pursuant thereto contained, contains or will contain any material misstatement of fact or omitted, omits or will omit to state any material fact necessary to make the statements therein, in the light of the circumstances under which they were, are or will be made, not misleading at the time such statement was made or deemed made. Additionally, any and all financial or business projections provided by Borrower to Lender shall be (i) provided in good faith and based on the most current data and information available to Borrower, (ii) the most current of such projections provided to Borrower's board of directors, and (iii) are based on reasonable assumptions not viewed as facts and that actual results during the period or periods covered by such projections and forecast may differ from the projected or forecasted results.

5.8 Tax Matters. Except as described on Schedule 5.8, (a) Borrower has filed all federal, state and local tax returns that it is required to file, (b) Borrower has duly paid or fully reserved for all taxes or installments thereof (including any interest or penalties) as and when due, which have or may become due pursuant to such returns, and (c) Borrower has paid or fully

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reserved for any tax assessment received by Borrower for the three (3) years preceding the Closing Date, if any (including any taxes being contested in good faith and by appropriate proceedings).

5.9 Intellectual Property Claims. Borrower is the sole owner of, or otherwise has the right to use, the Intellectual Property. Except as described on Schedule 5.9, (i) each of the material Copyrights, Trademarks and Patents is valid and enforceable, (ii) no material part of the Intellectual Property has been judged invalid or unenforceable, in whole or in part, and (iii) no claim has been made in writing to Borrower that any material part of the Intellectual Property violates the rights of any third party. Exhibit D is a true, correct and complete list of each of Borrower's Patents, registered Trademarks, registered Copyrights, and material agreements under which Borrower licenses Intellectual Property from third parties (other than shrink-wrap software licenses and other licenses for over-the-counter software), together with application or registration numbers, as applicable, owned by Borrower or any Subsidiary, in each case as of the Closing Date. Borrower is not in material breach of, nor has Borrower failed to perform any material obligations under, any of the foregoing contracts, licenses or agreements and, to Borrower's knowledge, no third party to any such contract, license or agreement is in material breach thereof or has failed to perform any material obligations thereunder.

5.10 Intellectual Property. Except as described on Schedule 5.10, Borrower has, or in the case of any proposed business, will have, all material rights with respect to Intellectual Property necessary in the operation or conduct of Borrower's business as currently conducted and proposed to be conducted by Borrower. Without limiting the generality of the foregoing, and in the case of Licenses, except for restrictions that are unenforceable under Division 9 of the UCC, Borrower has the right, to the extent required to operate Borrower's business, to freely transfer, license or assign Intellectual Property without condition, restriction or payment of any kind (other than license payments in the ordinary course of business) to any third party, and Borrower owns or has the right to use, pursuant to valid licenses, all software development tools, library functions, compilers and all other third-party software and other items that are necessary in the design, development, promotion, sale, license, manufacture, import, export, use or distribution of Borrower Products.

5.11 Borrower Products. Except as described on Schedule 5.11, no Intellectual Property owned by Borrower or Borrower Product has been or is subject to any actual or, to the knowledge of Borrower, threatened litigation, proceeding or outstanding decree, order, judgment, settlement agreement or stipulation that restricts in any material manner Borrower's use, transfer or licensing thereof or that may materially affect the validity, use or enforceability thereof. There is no decree, order, judgment, agreement, stipulation, arbitral award or other provision entered into in connection with any litigation or proceeding that obligates Borrower to grant licenses or ownership interest in any future Intellectual Property related to the operation or conduct of the business of Borrower or Borrower Products. Borrower has not received any written notice or claim, or, to the knowledge of Borrower, oral notice or claim, challenging or questioning Borrower's ownership in any Intellectual Property (or written notice of any claim challenging or questioning the ownership in any licensed Intellectual Property of the owner thereof) or suggesting that any third party has any claim of legal or beneficial ownership with respect thereto nor, to Borrower's knowledge, is there a reasonable basis for any such claim. To

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Borrower's knowledge, neither Borrower's use of its Intellectual Property nor the production and sale of Borrower Products infringes the Intellectual Property or other rights of others.

5.12 Financial Accounts. Exhibit E, as may be updated by the Borrower in a written notice provided to Lender after the Closing Date, is a true, correct and complete list of (a) all banks and other financial institutions at which Borrower or any Subsidiary maintains Deposit Accounts and (b) all institutions at which Borrower or any Subsidiary maintains an account holding Investment Property, and such exhibit correctly identifies the name, address and telephone number of each bank or other institution, the name in which the account is held, a description of the purpose of the account, and the complete account number therefor.

5.13 Employee Loans. Borrower has no outstanding loans to any employee, officer or director of the Borrower nor has Borrower guaranteed the payment of any loan made to an employee, officer or director of the Borrower by a third party.

5.14 Capitalization and Subsidiaries. Borrower's capitalization as of the Closing Date is set forth on Schedule 5.14 annexed hereto. Borrower does not own any stock, partnership interest or other securities of any Person, except for Permitted Investments. Attached as Schedule 5.14, as may be updated by Borrower in a written notice provided after the Closing Date, is a true, correct and complete list of each Subsidiary.

5.15 Centre of main interests and establishments. Borrower has its 'centre of main interests' (as that term is used in article 3(1) of The Council of the European Union Regulation No. 1346/2000 on Insolvency Proceedings) in the Netherlands.

## **SECTION 6. INSURANCE; INDEMNIFICATION**

6.1 Coverage. Borrower shall cause to be carried and maintained commercial general liability insurance, on an occurrence form, against risks customarily insured against in Borrower's line of business. Such risks shall include the risks of bodily injury, including death, property damage, personal injury, advertising injury, and contractual liability per the terms of the indemnification agreement found in Section 6.3. Borrower must maintain a minimum of \$1,000,000 of commercial general liability insurance for each occurrence and \$2,000,000 in the aggregate. Borrower has and agrees to maintain a minimum of \$2,000,000 of directors' and officers' insurance for each occurrence and \$5,000,000 in the aggregate. So long as there are any Secured Obligations outstanding, Borrower shall also cause to be carried and maintained insurance upon the Collateral, insuring against all risks of physical loss or damage howsoever caused, in an amount not less than the full replacement cost of the Collateral, provided that such insurance may be subject to standard exceptions and deductibles. Borrower shall also carry and maintain a fidelity insurance policy in an amount not less than \$100,000.

6.2 Certificates. Borrower shall deliver to Lender certificates of insurance that evidence Borrower's compliance with its insurance obligations in Section 6.1 and the obligations contained in this Section 6.2. Borrower's insurance certificate shall state Lender is an additional insured for commercial general liability, a loss payee for all risk property damage insurance, subject to the insurer's approval, a loss payee for fidelity insurance, and a loss payee for property insurance and additional insured for liability insurance for any future insurance that Borrower

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may acquire from such insurer, unless any right under the liability insurance is restricted from being pledged under Section 7:954(4) of the Dutch Civil Code. Attached to the certificates of insurance will be additional insured endorsements for liability and lender's loss payable endorsements for all risk property damage insurance and fidelity. Unless an Event of Default shall have occurred and be continuing, all insurance proceeds shall be paid or turned over to Borrower. All certificates of insurance will provide for a minimum of thirty (30) days advance written notice to Lender of cancellation or any other change adverse to Lender's interests. Any failure of Lender to scrutinize such insurance certificates for compliance is not a waiver of any of Lender's rights, all of which are reserved.

6.3 Indemnity. Borrower agrees to indemnify and hold Lender and its officers, directors, employees, agents, in-house attorneys, representatives and shareholders harmless from and against any and all claims, costs, expenses, damages and liabilities (including such claims, costs, expenses, damages and liabilities based on liability in tort, including strict liability in tort), including reasonable documented attorneys' fees and disbursements and other costs of investigation or defense (including those incurred upon any appeal), that may be instituted or asserted against or incurred by Lender or any such Person as the result of credit having been extended, suspended or terminated under this Agreement and the other Loan Documents or the administration of such credit, or in connection with or arising out of the transactions contemplated hereunder and thereunder, or any actions or failures to act in connection therewith, or arising out of the disposition or utilization of the Collateral, excluding in all cases claims resulting solely from Lender's gross negligence or willful misconduct. Borrower agrees to pay, and to save Lender harmless from, any and all liabilities with respect to, or resulting from any delay in paying, any and all excise, sales or other similar taxes (excluding taxes imposed on or measured by the net income of Lender) that may be payable or determined to be payable with respect to any of the Collateral or this Agreement.

## **SECTION 7. COVENANTS OF BORROWER**

Borrower agrees as follows:

(a) as soon as practicable (and in any event within 30 days) after the end of each month, unaudited interim and year-to-date financial statements as of the end of such month (prepared on a consolidated and consolidating basis, if applicable), including balance sheet and related statements of income and cash flows accompanied by a report detailing any material contingencies (including the commencement of any material litigation by or against Borrower) or any other occurrence that would reasonably be expected to have a Material Adverse Effect, all certified by Borrower’s Chief Executive Officer or Chief Financial Officer to the effect that they have been prepared in accordance with IFRS, except (i) for the absence of footnotes, (ii) that they are subject to normal year-end adjustments, and (iii) they do not contain certain non-cash items that are customarily included in quarterly and annual financial statements;

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(b) as soon as practicable (and in any event within 45 days) after the end of each calendar quarter, unaudited interim and year-to-date financial statements as of the end of such calendar quarter (prepared on a consolidated and consolidating basis, if applicable), including balance sheet and related statements of income and cash flows accompanied by a report detailing any material contingencies (including the commencement of any material litigation by or against Borrower) or any other occurrence that would reasonably be expected to have a Material Adverse Effect, certified by Borrower’s Chief Executive Officer or Chief Financial Officer to the effect that they have been prepared in accordance with IFRS, except (i) for the absence of footnotes, and (ii) that they are subject to normal year-end adjustments; as well as the most recent capitalization table for Borrower, including the weighted average exercise price of employee stock options;

(c) as soon as practicable (and in any event within one hundred fifty (150) days) after the end of each fiscal year, unqualified audited financial statements as of the end of such year (prepared on a consolidated and consolidating basis, if applicable), including balance sheet and related statements of income and cash flows, and setting forth in comparative form the corresponding figures for the preceding fiscal year, certified by a firm of independent certified public accountants selected by Borrower and reasonably acceptable to Lender, accompanied by any management report from such accountants;

(d) as soon as practicable (and in any event within 30 days) after the end of each month, a Compliance Certificate in the form of Exhibit F;

(e) promptly after the sending or filing thereof, as the case may be, copies of any proxy statements, financial statements or reports that US Borrower has made available to holders of its capital stock and copies of any regular, periodic and special reports or registration statements that US Borrower files with the Securities and Exchange Commission or any governmental authority that may be substituted therefor, or any national securities exchange, including;

(f) notify Lender in writing at least two (2) weeks in advance of the time and place of any regularly scheduled meeting of the board of Directors of Borrower (including without limitation telephone, conference call and video meetings). Borrower shall give Lender copies of all notices, minutes, consents and other materials the Borrower provides to its directors in connection with said meetings;

(g) Borrower at all times shall maintain cash and/or cash equivalents on deposit in a deposit or security account located in the United States that is subject to an Account Control Agreement of at least the lesser of (i) \$20,000,000 or (ii) 50% of all of the worldwide cash and cash equivalents of the Borrower;

(h) financial and business projections promptly following their approval by Borrower’s board of Directors, as well as budgets, operating plans and other financial information with respect to Borrower or its Subsidiaries reasonably requested by Lender;

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(i) Borrower shall not make any change in its (a) accounting policies or reporting practices except in accordance with IFRS, or (b) fiscal years or fiscal quarters. The fiscal year of Borrower shall end on December 31; and

(j) The executed Compliance Certificate may be sent via facsimile to Lender at (650) 473-9194 or via e-mail to BJadot@HTGC.com. All Financial Statements required to be delivered pursuant to clauses (a), (b) and (c) shall be sent via e-mail to financialstatements@herculestech.com with a copy to BJadot@HTGC.com and BBang@HTGC.com provided, that if e-mail is not available or sending such Financial Statements via e-mail is not possible, they shall be sent via facsimile to Lender at: (866) 468-8916, attention Chief Credit Officer.

7.2 Management Rights. Borrower shall permit any representative that Lender authorizes, including its attorneys and accountants, to inspect the Collateral and examine and make copies and abstracts of the books of account and records of Borrower at reasonable times and upon reasonable notice during normal business hours. In addition, any such representative shall have the right to meet with management and officers of Borrower to discuss such books of account and records. In addition, Lender shall be entitled at reasonable times and intervals to consult with and advise the management and officers of Borrower concerning significant business issues affecting Borrower. Such consultations shall not unreasonably interfere with Borrower’s business operations. The parties intend that the rights granted Lender shall constitute “management rights” within the meaning of 29 C.F.R Section 2510.3-101(d)(3) (ii), but that any advice, recommendations or participation by Lender with respect to any business issues shall not be deemed to give Lender, nor be deemed an exercise by Lender of, control over Borrower’s management or policies.

7.3 Further Assurances. Borrower shall from time to time execute, deliver and file, alone or with Lender, any financing statements, security agreements, collateral assignments, notices, control agreements, or other documents to perfect or give the highest priority to Lender’s Lien on the Collateral. Borrower shall from time to time procure any instruments or documents as may reasonably be requested by Lender, and take all further action that may be necessary or desirable, or that Lender may reasonably request, to perfect and protect the Liens granted hereby and thereby. In addition, and for such purposes only, Borrower hereby authorizes Lender to execute and deliver on behalf of Borrower and to file such financing statements, collateral assignments, notices, control agreements, security agreements and other documents necessary to grant, perfect and give the highest priority to Lender’s Lien on the Collateral without the signature of Borrower either in Lender’s name or in the name of Lender as agent and attorney-in-fact for Borrower. Borrower shall protect and defend Borrower’s title to the Collateral and Lender’s Lien thereon against all Persons claiming any interest adverse to Borrower or Lender other than Permitted Liens.

7.4 Indebtedness. Borrower shall not create, incur, assume, guarantee or be or remain liable with respect to any Indebtedness, or permit any Subsidiary so to do, other than Permitted Indebtedness, or prepay any Indebtedness or take any actions which impose on Borrower an obligation to prepay any Indebtedness, except for the conversion of Indebtedness into equity securities and the payment of cash in lieu of fractional shares in connection with such conversion.

7.5 Collateral. Borrower shall at all times keep the Collateral, the Intellectual Property and all other property and assets used in Borrower's business or in which Borrower now or hereafter holds any interest free and clear from any legal process or Liens whatsoever (except for Permitted Liens), and shall give Lender prompt written notice of any legal process affecting the Collateral, the Intellectual Property, such other property and assets, or any Liens thereon. Borrower shall cause its Subsidiaries to protect and defend such Subsidiary's title to its assets from and against all Persons claiming any interest adverse to such Subsidiary, and Borrower shall cause its Subsidiaries at all times to keep such Subsidiary's property and assets free and clear from any legal process or Liens whatsoever (except for Permitted Liens), and shall give Lender prompt written notice of any legal process affecting such Subsidiary's assets. Borrower shall not agree with any Person other than Lender not to encumber its property.

7.6 Investments. Borrower shall not directly or indirectly acquire or own, or make any Investment in or to any Person, or permit any of its Subsidiaries so to do, other than Permitted Investments.

7.7 Distributions. Borrower shall not, and shall not allow any Subsidiary to, (a) repurchase or redeem any class of stock or other equity interest other than pursuant to employee, director or consultant repurchase plans, stock option plans or agreements, restricted stock agreements or other similar agreements, provided, however, in each case the repurchase or redemption price does not exceed the original consideration paid for such stock or equity interest, or (b) declare or pay any cash dividend or make a cash distribution on any class of stock or other equity interest, except that a Subsidiary may pay dividends or make distributions to Borrower, or (c) lend money to any employees, officers or directors or guarantee the payment of any such loans granted by a third party in excess of \$250,000 in the aggregate or (d) waive, release or forgive any indebtedness owed by any employees, officers or directors in excess of \$250,000 in the aggregate.

7.8 Transfers. Except for Permitted Transfers, Borrower shall not voluntarily or involuntarily transfer, sell, lease, license, lend or in any other manner convey any equitable, beneficial or legal interest in any material portion of their assets.

7.9 Mergers or Acquisitions. Borrower shall not merge or consolidate, or permit any of its Subsidiaries to merge or consolidate, with or into any other business organization (other than mergers or consolidations of (i) a Subsidiary into Borrower, or (ii) of a Subsidiary which is not a Borrower into any Subsidiary or into Borrower, provided, in each case, that with respect to any merger into Borrower, Borrower is the surviving entity) or acquire, or permit any of its Subsidiaries to acquire, all or substantially all of the capital stock or property of another Person.

7.10 Taxes. Borrower and its Subsidiaries shall pay when due all taxes, fees or other charges of any nature whatsoever (together with any related interest or penalties) now or hereafter imposed or assessed against Borrower, Lender or the Collateral or upon Borrower's ownership, possession, use, operation or disposition thereof or upon Borrower's rents, receipts or earnings arising therefrom. Borrower shall file on or before the due date therefor all personal property tax returns in respect of the Collateral. Notwithstanding the foregoing, Borrower may contest, in good faith and by appropriate proceedings, taxes for which Borrower maintains adequate reserves therefor in accordance with IFRS.

7.11 Corporate Changes. Neither Borrower nor any Subsidiary shall change its corporate name, legal form or jurisdiction of formation without twenty (20) days' prior written notice to Lender. Neither Borrower nor any Subsidiary shall relocate its chief executive office or its principal place of business unless: (i) it has provided prior written notice to Lender; and (ii) such relocation shall be within the Netherlands. Neither Borrower nor any Subsidiary shall relocate any item of Collateral (other than (x) sales of movable assets in the ordinary course of business, (y) relocations of movable assets having an aggregate value of up to \$250,000 in any fiscal year, and (z) relocations of Collateral from a location described on Exhibit C to another location described on Exhibit C) unless (i) it has provided prompt written notice to Lender, (ii) such relocation is within the Netherlands and, (iii) if such relocation is to a third party bailee, it has delivered a bailee agreement in form and substance reasonably acceptable to Lender.

7.12 Deposit Accounts. Neither Borrower nor any Subsidiary shall maintain any Deposit Accounts (other than payroll, trust or escrow accounts), or accounts holding Investment Property, except with respect to which Lender has an Account Control Agreement and/or a first ranking right of pledge.

7.13 Subsidiaries. Borrower shall notify Lender of each Subsidiary formed subsequent to the Closing Date and, within 15 days of formation, shall cause any such Subsidiary to execute and deliver to Lender a Joinder Agreement.

7.14 Pensions. Borrower shall ensure that all pension schemes operated by or maintained for the benefit of members of the group and/or any of their employees are funded to the extent required by applicable law and regulations where failure to do so would be reasonably likely to have a Material Adverse Effect.

7.15 [Reserved].

## **SECTION 8. RIGHT TO INVEST**

8.1 Lender or its assignee or nominee shall have the right, in its discretion, to participate in any Subsequent Financing in an amount of up to Two Million Dollars (\$2,000,000) on the same terms, conditions and pricing afforded to others participating in any such Subsequent Financing.

## **SECTION 9. EVENTS OF DEFAULT**

The occurrence of any one or more of the following events shall be an Event of Default:

- 9.1 Payments. Borrower fails to pay any amount when due under this Agreement or any of the other Loan Documents; or
- 9.2 Covenants. Borrower breaches or defaults in the performance of any covenant or Secured Obligation under this Agreement, or any of the other Loan Documents, and (a) with respect to a default under any covenant under this Agreement (other than under Sections 6, 7.1(g), 7.5, 7.6, 7.7, 7.8 or 7.9) such default continues for more than fifteen (15) days after the earlier of the date on which (i) Lender has given notice of such default to Borrower and (ii)
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- Borrower has actual knowledge of such default or (b) with respect to a default under any of Sections 6, 7.1(g), 7.5, 7.6, 7.7, 7.8 or 7.9, the occurrence of such default; or
- 9.3 Material Adverse Effect. A circumstance has occurred that would reasonably be expected to have a Material Adverse Effect; or
- 9.4 Other Loan Documents. The occurrence of any default under any Loan Document and such default continues for more than ten (10) days after the earlier of (a) Lender has given notice of such default to Borrower, or (b) Borrower has actual knowledge of such default; or
- 9.5 Representations. Any material representation or warranty made by Borrower or uniQure Holdings in any Loan Document shall have been false or misleading in any material respect; or
- 9.6 Insolvency. Borrower (A) (i) shall make an assignment for the benefit of creditors; or (ii) shall be unable to pay its debts as they become due, or be unable to pay or perform under the Loan Documents, or shall become insolvent; or (iii) shall file a voluntary petition in bankruptcy; or (iv) shall file any petition, answer, or document seeking for itself any reorganization, arrangement, composition, readjustment, liquidation, dissolution or similar relief under any present or future statute, law or regulation pertinent to such circumstances; or (v) shall seek or consent to or acquiesce in the appointment of any trustee, receiver, or liquidator of Borrower or of all or any substantial part (i.e., 33-1/3% or more) of the assets or property of Borrower; or (vi) shall cease operations of its business as its business has normally been conducted, or terminate substantially all of its employees; (vii) Borrower or its directors or majority shareholders shall take any action initiating any of the foregoing actions described in clauses (i) through (vi); or (B) either (i) forty-five (45) days shall have expired after the commencement of an involuntary action against Borrower seeking reorganization, arrangement, composition, readjustment, liquidation, dissolution or similar relief under any present or future statute, law or regulation, without such action being dismissed or all orders or proceedings thereunder affecting the operations or the business of Borrower being stayed; or (ii) a stay of any such order or proceedings shall thereafter be set aside and the action setting it aside shall not be timely appealed; or (iii) Borrower shall file any answer admitting or not contesting the material allegations of a petition filed against Borrower in any such proceedings; or (iv) the court in which such proceedings are pending shall enter a decree or order granting the relief sought in any such proceedings; or (v) thirty (30) days shall have expired after the appointment, without the consent or acquiescence of Borrower, of any trustee, receiver or liquidator of Borrower or of all or any substantial part of the properties of Borrower without such appointment being vacated; or
- 9.7 Attachments; Judgments. Any portion of Borrower's assets is attached or seized, or a levy is filed against any such assets (and such attachment, seizure or levy is not lifted or released within 30 days), or a judgment or judgments (no longer subject to appeal) is/are entered for the payment of money, individually or in the aggregate, of at least \$250,000, or Borrower is enjoined or in any way prevented by court order from conducting any part of its business; or
- 9.8 Other Obligations. The occurrence of any default (beyond any applicable grace, appeal or cure periods) under any agreement or obligation of Borrower involving any

Indebtedness in excess of \$250,000, or the occurrence of any default by the Borrower under any agreement or obligation of Borrower that could reasonably be expected to have a Material Adverse Effect.

## **SECTION 10. REMEDIES**

- 10.1 General. Upon and during the continuance of any one or more Events of Default, (i) Lender may, at its option, accelerate and demand payment of all or any part of the Secured Obligations together with a Prepayment Charge and declare them to be immediately due and payable (provided, that upon the occurrence of an Event of Default of the type described in Section 9.6, all of the Secured Obligations shall automatically be accelerated and made due and payable, in each case without any further notice or act), and (ii) Lender may notify any of Borrower's account debtors to make payment directly to Lender, compromise the amount of any such account on Borrower's behalf and endorse Lender's name without recourse on any such payment for deposit directly to Lender's account. Lender may exercise all rights and remedies with respect to the Collateral under the Loan Documents or otherwise available to it under the laws of the Netherlands, the UCC and other applicable law, including the right to release, hold, sell, lease, liquidate, collect, realize upon, or otherwise dispose of all or any part of the Collateral and the right to occupy, utilize, process and commingle the Collateral.
- 10.2 Collection; Foreclosure. Unless otherwise agreed in the Collateral Documents, upon the occurrence and during the continuance of any Event of Default, Lender may, at any time or from time to time, apply, collect, liquidate, sell in one or more sales, lease or otherwise dispose of, any or all of the Collateral, in its then condition or following any commercially reasonable preparation or processing, in such order as Lender may elect. Any such sale may be made either at public or private sale at its place of business or elsewhere. Borrower agrees that any such public or private sale may occur upon ten (10) calendar days' prior written notice to Borrower. Lender may require Borrower to assemble the Collateral and make it available to Lender at a place designated by Lender that is reasonably convenient to Lender and Borrower. The proceeds of any sale, disposition or other realization upon all or any part of the Collateral shall be applied by Lender in the following order of priorities:

First, to Lender in an amount sufficient to pay in full Lender's costs and professionals' and advisors' fees and expenses as described in Section 11.11;

Second, to Lender in an amount equal to the then unpaid amount of the Secured Obligations (including principal, interest, and the Default Rate interest), in such order and priority as Lender may choose in its sole discretion; and

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11.6 Survival. All agreements, representations and warranties contained in this Agreement and the other Loan Documents or in any document delivered pursuant hereto or thereto shall be for the benefit of Lender and shall survive the execution and delivery of this Agreement and the expiration or other termination of this Agreement.

11.7 Successors and Assigns. The provisions of this Agreement and the other Loan Documents shall inure to the benefit of and be binding on Borrower and its permitted assigns (if any). Borrower shall not assign its obligations under this Agreement or any of the other Loan Documents without Lender's express prior written consent, and any such attempted assignment shall be void and of no effect. Lender may assign, transfer, or endorse its rights hereunder and under the other Loan Documents without prior notice to Borrower, and all of such rights shall inure to the benefit of Lender's successors and assigns.

11.8 Governing Law. This Agreement and the other Loan Documents shall be governed by, and construed and enforced in accordance with, the laws of the Netherlands.

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11.9 Jurisdiction. The courts (*Rechtbank*) of Amsterdam, the Netherlands, subject to ordinary appeal and final appeal shall have exclusive jurisdiction to hear and determine any suit, action or proceeding and to settle any disputes arising out of or in connection with this Agreement and the other Loan Documents (including a dispute regarding the existence, validity or termination of this Agreement or the consequences of its nullity) and, for such purposes, each of the parties hereto irrevocably submits to the exclusive jurisdiction of such courts. This Section is for the benefit of the Lender only. As a result, the Lender may take proceedings relating to a dispute in any other courts with jurisdiction. To the extent allowed by law, the Lender may take concurrent proceedings in any number of jurisdictions.

11.10 Professional Fees. Borrower promises to pay Lender's documented out-of-pocket fees and expenses necessary to finalize the loan documentation, including but not limited to reasonable documented attorneys' fees, UCC searches, filing costs, and other miscellaneous expenses. In addition, Borrower promises to pay any and all reasonable documented attorneys' and other professionals' fees and expenses (including fees and expenses of in-house counsel) incurred by Lender after the Closing Date in connection with or related to: (a) the Loan; (b) the administration, collection, or enforcement of the Loan; (c) the amendment or modification of the Loan Documents; (d) any waiver, consent, release, or termination under the Loan Documents; (e) the protection, preservation, sale, lease, liquidation, or disposition of Collateral or the exercise of remedies with respect to the Collateral; (f) any legal, litigation, administrative, arbitration, or out of court proceeding in connection with or related to Borrower or the Collateral, and any appeal or review thereof; and (g) any bankruptcy, restructuring, reorganization, assignment for the benefit of creditors, workout, foreclosure, or other action related to Borrower, the Collateral, the Loan Documents, including representing Lender in any adversary proceeding or contested matter commenced or continued by or on behalf of Borrower's estate, and any appeal or review thereof.

11.11 Confidentiality. Lender acknowledges that all financial statements provided to Lender by Borrower and certain items of Collateral and information provided to Lender by Borrower are confidential and proprietary information of Borrower, if and to the extent such information either (x) is marked as confidential by Borrower at the time of disclosure, or (y) should reasonably be understood to be confidential (the "Confidential Information"). Accordingly, Lender agrees that any Confidential Information it may obtain in the course of acquiring, administering, or perfecting Lender's security interest in the Collateral shall not be disclosed to any other person or entity in any manner whatsoever, in whole or in part, without the prior written consent of Borrower, except that Lender may disclose any such information: (a) to its own directors, officers, employees, accountants, counsel and other professional advisors and to its affiliates if Lender in its sole discretion determines that any such party should have access to such information in connection with such party's responsibilities in connection with the Loan or this Agreement and, provided that such recipient of such Confidential Information either (i) agrees to be bound by the confidentiality provisions of this paragraph or (ii) is otherwise subject to confidentiality restrictions that reasonably protect against the disclosure of Confidential Information; (b) if such information is generally available to the public; (c) if required or appropriate in any report, statement or testimony submitted to any governmental authority having or claiming to have jurisdiction over Lender; (d) if required or appropriate in response to any summons or subpoena or in connection with any litigation, to the extent permitted or deemed advisable by Lender's counsel; (e) to comply with any legal requirement or law applicable to Lender; (f) to the extent reasonably necessary in connection with the exercise of any right or

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remedy under any Loan Document, including Lender's sale, lease, or other disposition of Collateral after the occurrence and during the continuance of an Event of Default; (g) to any participant or assignee of Lender or any prospective participant or assignee; provided, that such participant or assignee or prospective participant or assignee agrees in writing to be bound by this Section prior to disclosure; or (h) otherwise with the prior consent of Borrower; provided, that any disclosure made in violation of this Agreement shall not affect the obligations of Borrower or any of its affiliates or any guarantor under this Agreement or the other Loan Documents.

11.12 Assignment of Rights. Borrower acknowledges and understands that Lender may sell and assign all or part of its interest hereunder and under the Loan Documents to any person or entity (an "Assignee"). After such assignment the term "Lender" as used in the Loan Documents shall mean and include such Assignee, and such Assignee shall be vested with all rights, powers and remedies of Lender hereunder with respect to the interest so assigned; but with respect to any such interest not so transferred, Lender shall retain all rights, powers and remedies hereby given. No such assignment by Lender shall relieve Borrower of any of its obligations hereunder. Lender agrees that in the event of any transfer by it of the Note(s) (if any), it will endorse thereon a notation as to the portion of the principal of the Note(s), which shall have been paid at the time of such transfer and as to the date to which interest shall have been last paid thereon.

11.13 Revival of Secured Obligations. This Agreement and the Loan Documents shall remain in full force and effect and continue to be effective if any petition is filed by or against Borrower for liquidation or reorganization, if Borrower becomes insolvent or makes an assignment for the benefit of creditors, if a receiver or trustee is appointed for all or any significant part of Borrower's assets, or if any payment or transfer of Collateral is recovered from Lender. The Loan Documents and the Secured Obligations and Collateral security shall continue to be effective, or shall be revived or reinstated, as the case may be, if at any time payment and performance of the Secured Obligations or any transfer of Collateral to Lender, or any part thereof is rescinded, avoided or avoidable, reduced in amount, or must otherwise be restored or returned by, or is recovered from, Lender or by any obligee of the Secured Obligations, whether as a "voidable preference," "fraudulent conveyance," or otherwise, all as though such payment, performance, or transfer of Collateral had not been made. In the event that any payment, or any part thereof, is rescinded, reduced, avoided, avoidable, restored, returned, or recovered, the Loan Documents and the Secured Obligations shall be deemed, without any further action or documentation, to have been revived and reinstated except to the extent of the full, final, and indefeasible payment to Lender in Cash.

11.14 Counterparts. This Agreement and any amendments, waivers, consents or supplements hereto may be executed in any number of counterparts, and by different parties hereto in separate counterparts, each of which when so delivered shall be deemed an original, but all of which

counterparts shall constitute but one and the same instrument.

11.15 Publicity.

(a) Borrower consents to the publication and use by Lender and any of its member businesses and affiliates of (i) Borrower's name (including a brief description of the

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relationship between Borrower and Lender) and logo for use on Lender's website and as required for the purposes of filings with or reports to governmental authorities required by law, and (ii) after review and approval by Borrower (a) Borrower's name and a hyperlink to Borrower's web site, separately or together, in written and oral presentations, advertising, promotional and marketing materials, client lists, public relations materials or on its web site (together, the "Lender Publicity Materials"); (b) the names of officers of Borrower in the Lender Publicity Materials; and (c) Borrower's name, trademarks or servicemarks in any news release concerning Lender.

(b) Neither Borrower nor any of its member businesses and affiliates shall, without Lender's consent, publicize or use, for any purpose other than filings with or reports to governmental authorities required by law and the rules of any applicable securities commission or securities exchange, (i) Lender's name (including a brief description of the relationship between Borrower and Lender), logo or hyperlink to Lender's web site, separately or together, in written and oral presentations, advertising, promotional and marketing materials, client lists, public relations materials or on its web site (together, the "Borrower Publicity Materials"); (ii) the names of officers of Lender in the Borrower Publicity Materials; and (iii) Lender's name, trademarks, servicemarks in any news release concerning Borrower.

11.16 Existing Loan and Security Agreement Amended and Restated. Upon satisfaction of the conditions precedent to the effectiveness of this Agreement, (a) this Agreement shall amend and restate the Existing Loan and Security Agreement in its entirety (except to the extent that definitions from the Existing Loan and Security Agreement are incorporated herein by reference) and (b) the rights and obligations of the parties under the Existing Loan and Security Agreement shall be subsumed within, and be governed by, this Agreement; provided, however, that the Borrower hereby agrees that all Secured Obligations of the Borrower under, and as defined in, the Existing Loan and Security Agreement and the other Loan Documents shall remain outstanding, shall constitute continuing Secured Obligations secured by the Collateral, and this Agreement shall not be deemed to evidence or result in a novation or repayment and re-borrowing of such obligations and other liabilities. Borrower hereby acknowledges and reaffirms each and every Loan Document entered into in connection with the Existing Loan and Security Agreement and acknowledges that each such Loan Document remains in full force and effect and enforceable against Borrower in accordance with its respective terms after giving effect to the execution and delivery of this Agreement without further action by Lender, Borrower or any other Person. All reference to the "Loan and Security Agreement" in each such Loan Document shall be deemed to be a reference to this Agreement.

(SIGNATURES TO FOLLOW)

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IN WITNESS WHEREOF, Borrower and Lender have duly executed and delivered this Loan and Security Agreement as of the day and year first above written.

BORROWER:

UNIQUE BIOPHARMA B.V.

Signature: /s/ JÖRN ALDAG

Print Name: Jörn Aldag

Title: CEO

UNIQUE IP B.V.

Signature: /s/ JÖRN ALDAG

Print Name: Jörn Aldag

Title: CEO

UNIQUE N.V. (formerly uniQure B.V.), solely a party hereto for purposes of Section 3.1(a)

Signature: /s/ JÖRN ALDAG

Print Name: Jörn Aldag

Title: CEO

UNIQUE RESEARCH B.V.\*

Signature: /s/ JÖRN ALDAG



Print Name: Jörn Aldag

Title: CEO

UNIQUE ASSAY DEVELOPMENT B.V.\*

Signature: /s/ JÖRN ALDAG

Print Name: Jörn Aldag

Title: CEO

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UNIQUE QA B.V.\*

Signature: /s/ JÖRN ALDAG

Print Name: Jörn Aldag

Title: CEO

UNIQUE PROCESS DEVELOPMENT B.V.\*

Signature: /s/ JÖRN ALDAG

Print Name: Jörn Aldag

Title: CEO

\* Wholly-owned subsidiary of uniQure Biopharma B.V.

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UNIQUE MANUFACTURING B.V.\*

Signature: /s/ JÖRN ALDAG

Print Name: Jörn Aldag

Title: CEO

UNIQUE NON CLINICAL B.V.\*

Signature: /s/ JÖRN ALDAG

Print Name: Jörn Aldag

Title: CEO

UNIQUE CLINICAL B.V.\*

Signature: /s/ JÖRN ALDAG

Print Name: Jörn Aldag

Title: CEO

UNIQUE, INC.

Signature: /s/ JÖRN ALDAG

Print Name: Jörn Aldag

Title: CEO

LENDER:

HERCULES TECHNOLOGY GROWTH CAPITAL, INC.

Signature: /s/ BEN BANG

Print Name: Ben Bang

Title: Senior Counsel

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\* Wholly-owned subsidiary of uniQure Biopharma B.V.

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Table of Addenda, Exhibits and Schedules

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Exhibit B:	Note
Exhibit C:	Name, Locations, and Other Information for Borrower
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EXHIBIT A

ADVANCE REQUEST

To: Lender: \_\_\_\_\_ Date: \_\_\_\_\_, 201

Hercules Technology Growth Capital, Inc.  
400 Hamilton Avenue, Suite 310  
Palo Alto, CA 94301  
Facsimile: 650-473-9194  
Attn:

UNIQUE BIOPHARMA B.V., a (“uniQure”), (ii) UNIQUE IP B.V., a (“uniQure IP”), (iii) each of the subsidiaries of uniQure identified on the Schedule 1 to the Agreement hereinafter referred to and the signature pages thereof (uniQure, uniQure IP and such subsidiaries are hereinafter collectively referred to as “**Borrower**”) (“Borrower”) hereby requests from Hercules Technology Growth Capital, Inc. (“Lender”) an Advance in the amount of \_\_\_\_\_ Dollars (\$) on \_\_\_\_\_, \_\_\_\_\_ (the “Advance Date”) pursuant to the Loan and Security Agreement between Borrower and Lender (the “Agreement”). Capitalized words and other terms used but not otherwise defined herein are used with the same meanings as defined in the Agreement.

Please:

(a) Issue a check payable to Borrower

or

(b) Wire Funds to Borrower's account

Bank:  
Address:

ABA Number:  
Account Number:  
Account Name:

Borrower represents that the conditions precedent to the Advance set forth in the Agreement are satisfied and shall be satisfied upon the making of such Advance, including but not limited to: (i) that no event that has had or could reasonably be expected to have a Material Adverse Effect has occurred and is continuing; (ii) that the representations and warranties set forth in the Agreement and in the Warrant Agreement are and shall be true and correct in all material respects on and as of the Advance Date with the same effect as though made on and as of such date, except to the extent such representations and warranties expressly relate to an earlier date; (iii) that Borrower is in compliance with all the terms and provisions set forth in each Loan Document on its part to be observed or performed; and (iv) that as of the Advance

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Date, no fact or condition exists that would (or would, with the passage of time, the giving of notice, or both) constitute an Event of Default under the Loan Documents. Borrower understands and acknowledges that Lender has the right to review the financial information supporting this representation and, based upon such review in its sole discretion, Lender may decline to fund the requested Advance.

Borrower hereby represents that Borrower's corporate status and locations have not changed since the date of the Agreement or, if the Attachment to this Advance Request is completed, are as set forth in the Attachment to this Advance Request.

Borrower agrees to notify Lender promptly before the funding of the Loan if any of the matters which have been represented above shall not be true and correct on the Borrowing Date and if Lender has received no such notice before the Advance Date then the statements set forth above shall be deemed to have been made and shall be deemed to be true and correct as of the Advance Date.

Executed as of [ ], 2014.

BORROWER:

UNIQUE BIOPHARMA B.V.

Signature: \_\_\_\_\_

Print Name: \_\_\_\_\_

Title: \_\_\_\_\_

UNIQUE IP B.V.

Signature: \_\_\_\_\_

Print Name: \_\_\_\_\_

Title: \_\_\_\_\_

UNIQUE RESEARCH B.V.

Signature: \_\_\_\_\_

Print Name: \_\_\_\_\_

Title: \_\_\_\_\_

UNIQUE ASSAY DEVELOPMENT B.V.

Signature: \_\_\_\_\_

Print Name: \_\_\_\_\_

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Title: \_\_\_\_\_

UNIQUE QA B.V.

Signature: \_\_\_\_\_

Print Name: \_\_\_\_\_

Title: \_\_\_\_\_

UNIQUE PROCESS DEVELOPMENT B.V.

Signature: \_\_\_\_\_

Print Name: \_\_\_\_\_

Title: \_\_\_\_\_

UNIQUE MANUFACTURING B.V.

Signature: \_\_\_\_\_

Print Name: \_\_\_\_\_

Title: \_\_\_\_\_

UNIQUE NON CLINICAL B.V.

Signature: \_\_\_\_\_

Print Name: \_\_\_\_\_

Title: \_\_\_\_\_

UNIQUE CLINICAL B.V.

Signature: \_\_\_\_\_

Print Name: \_\_\_\_\_

Title: \_\_\_\_\_

UNIQUE, INC.

Signature: \_\_\_\_\_

Print Name: \_\_\_\_\_

Title: \_\_\_\_\_

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#### **ATTACHMENT TO ADVANCE REQUEST**

Dated:\_\_\_\_\_

Borrower hereby represents and warrants to Lender that Borrower’s current name and organizational status is as follows:

Name:

Type of organization:

State of organization:

Organization file number:

Borrower hereby represents and warrants to Lender that the street addresses, cities, states and postal codes of its current locations are as follows:

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#### **EXHIBIT B**

#### **AMENDED AND RESTATED PROMISSORY NOTE**

FOR VALUE RECEIVED, (i) UNIQUE BIOPHARMA B.V., a private limited liability company incorporated and existing under the laws of the Netherlands, having its corporate seat at Amsterdam, the Netherlands and registered at the trade register of the Chamber of Commerce for Amsterdam under number 34275365 (“**uniQure**”), (ii) UNIQUE IP B.V., a private limited liability company incorporated and existing under the laws of the Netherlands, having its corporate seat at Amsterdam, the Netherlands and registered at the trade register of the Chamber of Commerce for Amsterdam under number 34275369 (“**uniQure IP**”), (iii) each of the subsidiaries of uniQure identified on the signature page hereof (uniQure, uniQure IP and such subsidiaries are hereinafter collectively referred to as “**Borrower**”) hereby promises to pay to the order of Hercules Technology Growth Capital, Inc., a Maryland corporation (the “Lender”) or the holder of this Amended and Restated Promissory Note (this “Promissory Note”) at 400 Hamilton Avenue, Suite 310, Palo Alto, CA 94301 or such other place of payment as the holder of this Promissory Note may specify from time to time in writing, in lawful money of the United States of America, the principal amount of Twenty Million Dollars (\$20,000,000) or such other principal amount as Lender has advanced to Borrower, together with interest at a floating rate per annum equal to the greater of either (a) ten and one quarter of one percent (10.25%), or (b) the sum of (1) ten and one quarter of one percent (10.25%), plus (2) the Prime Rate minus five and one quarter of one percent (5.25%), based upon a year consisting of 360 days, with interest computed daily based on the actual number of days in each month.

This Promissory Note is the Note referred to in, and is executed and delivered in connection with, that certain Amended and Restated Loan and Security Agreement dated June 10, 2014, by and between Borrower and Lender (as the same may from time to time be amended, modified or supplemented in accordance with its terms, the “Loan Agreement”), and is entitled to the benefit and security of the Loan Agreement and the other Loan Documents (as defined in the Loan Agreement), to which reference is made for a statement of all of the terms and conditions thereof. All payments shall be made in accordance with the Loan Agreement. All terms defined in the Loan Agreement shall have the same definitions when used herein, unless otherwise defined herein. An Event of Default under the Loan Agreement shall constitute a default under this Promissory Note.

Borrower agrees to make all payments under this Promissory Note without setoff, recoupment or deduction and regardless of any counterclaim or defense. This Promissory Note has been negotiated and delivered to Lender and is payable in the State of California. This Promissory Note shall be governed by and construed and enforced in accordance with, the laws of the Netherlands, excluding any conflicts of law rules or principles that would cause the application of the laws of any other jurisdiction.

BORROWER:  
  
UNIQUE BIOPHARMA B.V.

	Signature:	_____
	Print Name:	_____
	Title:	_____
	UNIQUE IP B.V.	
	Signature:	_____
	Print Name:	_____
	Title:	_____
	UNIQUE RESEARCH B.V.	
	Signature:	_____
	Print Name:	_____
	Title:	_____
	UNIQUE ASSAY DEVELOPMENT B.V.	
	Signature:	_____
	Print Name:	_____
	Title:	_____
	UNIQUE QA B.V.	
	Signature:	_____
	Print Name:	_____
	Title:	_____
	UNIQUE PROCESS DEVELOPMENT B.V.	

Signature: \_\_\_\_\_  
Print Name: \_\_\_\_\_  
Title: \_\_\_\_\_

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UNIQUE MANUFACTURING B.V.

Signature: \_\_\_\_\_  
Print Name: \_\_\_\_\_  
Title: \_\_\_\_\_

UNIQUE NON CLINICAL B.V.

Signature: \_\_\_\_\_  
Print Name: \_\_\_\_\_  
Title: \_\_\_\_\_

UNIQUE CLINICAL B.V.

Signature: \_\_\_\_\_  
Print Name: \_\_\_\_\_  
Title: \_\_\_\_\_

UNIQUE, INC.

Signature: \_\_\_\_\_  
Print Name: \_\_\_\_\_  
Title: \_\_\_\_\_

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## EXHIBIT C

### NAME, LOCATIONS, AND OTHER INFORMATION FOR BORROWER

1. Borrower represents and warrants to Lender that Borrower's current name and organizational status as of each applicable Advance Date is as follows:

Name:	uniQure biopharma B.V.
Type of organization:	private limited liability company ( <i>besloten vennootschap met beperkte aansprakelijkheid</i> )
Country of organization:	The Netherlands
Organization file number:	54385229

2. Borrower represents and warrants to Lender that for five (5) years prior to the Closing Date, Borrower did not do business under any other name or organization or form except the following:

Name:	AMT biopharma B.V.
Used during dates of:	28 December 2011 – 5 April 2012
Type of Organization:	private limited liability company
Country of organization:	The Netherlands
Organization file Number:	54385229
Borrower's fiscal year ends on	31 December
Borrower's employer tax identification number is:	851284334

3. Borrower represents and warrants to Lender that its chief executive office is located at Meibergdreef 61, 1105 BA Amsterdam, the Netherlands.

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#### EXHIBIT D

#### BORROWER'S PATENTS, TRADEMARKS, COPYRIGHTS AND LICENSES

[PROVIDED SEPARATELY]

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#### EXHIBIT E

#### BORROWER'S DEPOSIT ACCOUNTS AND INVESTMENT ACCOUNTS

Bank address details of all the Dutch entity accounts:

Rabobank Amsterdam  
Corporate Banking  
Mondriaantoren, Amstelplein 8  
1096 BC Amsterdam  
The Netherlands

Bank address details of all the US accounts:

Rabobank N.A.  
301 Main Street  
Salinas, California 93901

Legal entity/account owner	IBAN
Uniqure N.V.	NL68 RABO 0168 4445 69
Uniqure, Inc. (operational account)	Rabobank NA 448387300
Uniqure, Inc. (checking account)	Rabobank NA 533386171
Uniqure Biopharma B.V.	NL02 RABO 0112 6530 06
Uniqure Biopharma B.V. (operational account)	Rabobank NA 322116245
Uniqure Biopharma B.V. (money market account)	Rabobank NA 291372402
Uniqure Biopharma BV (savings acct)	NL87 RABO 1095 4145 42
Uniqure Biopharma BV (savings acct)	NL19 RABO 1095 7018 19
Uniqure Assay Development B.V.	NL88 RABO 0166 9831 95
Uniqure Clinical B.V.	NL98 RABO 0166 9832 09
Uniqure Manufacturing B.V.	NL76 RABO 0166 9832 17
Uniqure Non Clinical B.V.	NL54 RABO 0166 9832 25
Uniqure Process Development B.V.	NL32 RABO 0166 9832 33
Uniqure QA B.V.	NL10 RABO 0166 9832 41
Uniqure Research B.V.	NL05 RABO 0167 7428 41

Legal entity/account owner	IBAN
Uniqure Biopharma B.V.	NL02 RABO 0112 6530 06

Legal entity/account owner	IBAN
Uniqure Biopharma B.V.	NL02 RABO 0112 6530 06

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#### EXHIBIT F

#### COMPLIANCE CERTIFICATE

Hercules Technology Growth Capital, Inc.  
400 Hamilton Avenue, Suite 310  
Palo Alto, CA 94301

Reference is made to that certain Amended and Restated Loan and Security Agreement dated June , 2014 and all ancillary documents entered into in connection with such Loan and Security Agreement all as may be amended from time to time, (hereinafter referred to collectively as the "Loan Agreement") between Hercules Technology Growth Capital, Inc. as Lender and (i) UNIQURE BIOPHARMA B.V., a ("uniQure"),

(ii) UNIQURE IP B.V., a (“uniQure IP”), (iii) each of the subsidiaries of uniQure (uniQure, uniQure IP and such subsidiaries are hereinafter collectively referred to as “Borrower”), as Borrower. All capitalized terms not defined herein shall have the same meaning as defined in the Loan Agreement.

The undersigned is an Officer of the Borrower, knowledgeable of all Borrower’s financial matters, and is authorized to provide certification of information regarding the Borrower; hereby certifies that in accordance with the terms and conditions of the Loan Agreement, the Borrower is in compliance for the period ending of all covenants, conditions and terms and hereby reaffirms that all representations and warranties contained therein are true and correct in all material respects on and as of the date of this Compliance Certificate with the same effect as though made on and as of such date, except to the extent such representations and warranties expressly relate to an earlier date, after giving effect in all cases to any standard(s) of materiality contained in the Loan Agreement as to such representations and warranties. Attached are the required documents supporting the above certification. The undersigned further certifies that these are prepared in accordance with IFRS (except for the absence of footnotes with respect to unaudited financial statement and subject to normal year-end adjustments) and are consistent from one period to the next except as explained below.

REPORTING REQUIREMENT REQUIRED CHECK IF ATTACHED

Interim Financial Statements	Monthly within 30 days
Interim Financial Statements	Quarterly within 45 days
Audited Financial Statements	FYE within 150 days

Very Truly Yours,

UNIQURE BIOPHARMA B.V.

By: \_\_\_\_\_

Name: \_\_\_\_\_

Title: \_\_\_\_\_

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Its: \_\_\_\_\_

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EXHIBIT G

FORM OF JOINDER AGREEMENT

This Joinder Agreement (the “Joinder Agreement”) is made and dated as of [ ], 20[ ], and is entered into by and between , a corporation (“Subsidiary”), and HERCULES TECHNOLOGY GROWTH CAPITAL, INC., a Maryland corporation, as a Lender.

RECITALS

A. Subsidiary’s Affiliates, (i) UNIQURE BIOPHARMA B.V., a (“uniQure”), (ii) UNIQURE IP B.V., a (“uniQure IP”), (iii) each of the subsidiaries of uniQure (uniQure, uniQure IP and such subsidiaries are hereinafter collectively referred to as “Borrower”) have entered into that certain Amended and Restated Loan and Security Agreement dated June , 2014, with Lender, as such agreement may be amended (the “Loan Agreement”), together with the other agreements executed and delivered in connection therewith;

B. Subsidiary acknowledges and agrees that it will benefit both directly and indirectly from Borrower’s execution of the Loan Agreement and the other agreements executed and delivered in connection therewith;

AGREEMENT

NOW THEREFORE, Subsidiary and Lender agree as follows:

- The recitals set forth above are incorporated into and made part of this Joinder Agreement. Capitalized terms not defined herein shall have the meaning provided in the Loan Agreement.
- By signing this Joinder Agreement, Subsidiary shall be bound by the terms and conditions of the Loan Agreement the same as if it were the Borrower (as defined in the Loan Agreement) under the Loan Agreement, mutatis mutandis, provided however, that Lender shall have no duties, responsibilities or obligations to Subsidiary arising under or related to the Loan Agreement or the other agreements executed and delivered in connection therewith. Rather, to the extent that Lender has any duties, responsibilities or obligations arising under or related to the Loan Agreement or the other agreements executed and delivered in connection therewith, those duties, responsibilities or obligations shall flow only to Borrower and not to Subsidiary or any other person or entity. By way of example (and not an exclusive list): (a) Lender’s providing notice to Borrower in accordance with the Loan Agreement or as otherwise agreed between Borrower and Lender shall be deemed provided to Subsidiary; (b) a Lender’s providing an Advance to Borrower shall be deemed an Advance to Subsidiary; and (c) Subsidiary shall have no right to request an Advance or make any other demand on Lender.



## [SIGNATURE PAGE TO JOINDER AGREEMENT]

SUBSIDIARY:

\_\_\_\_\_.

By:  
Name:  
Title:  
Address:

Telephone:  
Facsimile:

HERCULES TECHNOLOGY GROWTH CAPITAL, INC.

By: \_\_\_\_\_  
Name: \_\_\_\_\_  
Title: \_\_\_\_\_

Address:  
400 Hamilton Ave., Suite 310  
Palo Alto, CA 94301  
Facsimile: 650-473-9194  
Telephone: 650-289-3060

## EXHIBIT H

## ACH DEBIT AUTHORIZATION AGREEMENT

Hercules Technology Growth Capital, Inc.  
400 Hamilton Avenue, Suite 310  
Palo Alto, CA 94301

Re: Amended and Restated Loan and Security Agreement dated June , 2014 between (i) UNIQUE BIOPHARMA B.V., a (“**uniQure**”), (ii) UNIQUE IP B.V., a (“**uniQure IP**”), (iii) each of the subsidiaries of uniQure (uniQure, uniQure IP and such subsidiaries are hereinafter collectively referred to as “**Borrower**”) and Hercules Technology Growth Capital, Inc. (“**Lender**”) (the “**Agreement**”)

In connection with the above referenced Agreement, Borrower hereby authorizes the Lender to initiate debit entries for the periodic payments due under the Agreement to t Borrower’s account indicated below. Borrower authorizes the depository institution named below to debit to such account.

DEPOSITORY NAME

BRANCH

CITY

STATE AND ZIP CODE

TRANSIT/ABA NUMBER

ACCOUNT NUMBER

This authority will remain in full force and effect so long as any amounts are due under the Agreement.

\_\_\_\_\_  
(Borrower)(Please Print)

By: \_\_\_\_\_  
Date: \_\_\_\_\_

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

Execution Copy  
April 6, 2015

## COLLABORATION AND LICENSE AGREEMENT

**THIS COLLABORATION AND LICENSE AGREEMENT** (this “**Agreement**”) is made and entered into as of April 6, 2015 (the “**Signing Date**”) by and between **UNIQURE BIOPHARMA B.V.**, a corporation organized under the laws of the Netherlands, having its principal place of business at Meibergdreef 61, 1105 BA Amsterdam, The Netherlands (“**uniQure**”), and **BRISTOL-MYERS SQUIBB COMPANY**, a Delaware corporation headquartered at 345 Park Avenue, New York, New York, USA 10154. uniQure and BMS are sometimes referred to herein individually as a “**Party**” and collectively as the “**Parties**”.

### RECITALS

**Whereas**, BMS is a biopharmaceutical company engaged in the research, development, manufacture and commercialization of human therapeutic products.

**Whereas**, uniQure is a biopharmaceutical company that, together with its Affiliates, owns or otherwise controls certain adeno-associated virus (AAV) based technology for Gene Therapy products.

**Whereas**, uniQure and BMS desire to collaborate in the performance of a Research Program for the purpose of discovery and preclinical development of certain Collaboration Targets and related Target Therapeutics suitable for development for Cardiovascular Diseases and other therapeutic indications and uses, with the objective of identifying one or more Therapeutics for BMS to advance into human clinical trials, in accordance with the terms and conditions set forth in this Agreement.

**Whereas**, BMS will have exclusive rights and will be solely responsible for the clinical development and commercialization of Therapeutics and Products worldwide, in accordance with the terms and conditions set forth in this Agreement.

**Now Therefore**, in consideration of the foregoing premises and the mutual promises, covenants and conditions contained in this Agreement, the Parties agree as follows.

### 1. DEFINITIONS

As used in this Agreement, the terms with initial letters capitalized, whether used in the singular or plural form, shall have the meanings set forth in this Article 1 or, if not listed below, the meanings designated in places throughout this Agreement.

**1.1** “**AAA**” has the meaning set forth in Section 16.2(b).

**1.2** “**AAA Rules**” has the meaning set forth in Section 16.2(b).

**1.3** “**Additional Rights**” has the meaning set forth in Section 7.8(a).

**1.4** “**Affiliate**” means, with respect to a particular Person, another Person that controls, is controlled by or is under common control with such Person. For the purposes of this definition, the term “control” (including, with correlative meaning, the terms “controlled by” or “under common control with”) means the actual power, either directly or indirectly through one or more intermediaries, to direct or cause the direction of the management and policies of such entity, whether by the ownership of more than fifty percent (50%) of the voting stock of such entity, or by contract or otherwise.

**1.5** “**Alliance Manager**” has the meaning set forth in Section 2.2.

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**1.6** “**API**” means active pharmaceutical ingredient.

**1.7** “**Applicable Law**” means any applicable supranational, federal, state, local or foreign law, statute, ordinance or principle of common law, or any rule, regulation, standard, judgment, order, writ, injunction, decree, arbitration award, agency guidelines or other requirement, license or permit of any Governmental Authority.

**1.8** “**Back-up Therapeutic**” means a Therapeutic with [\*\*] and [\*\*] as a [\*\*] but a different [\*\*] (including a [\*\*] with [\*\*] to the [\*\*]) that is being Developed as a [\*\*] in the same indication in case of [\*\*] with the [\*\*] (which may result in a termination of further Development of the [\*\*]).

**1.9** “**Bankrupt Party**” has the meaning set forth in Section 17.3(a).

**1.10** “**BLA**” means a Biologics License Application, for which Regulatory Approval by the FDA is required to market a Product in the U.S.

**1.11** “**BLA Approval**” means, with respect to a Product, receipt of Regulatory Approval of a BLA by the FDA for such Product in the U.S.

**1.12** “**BLA Filing**” means the acceptance by the FDA of the filing of a BLA with the FDA for the applicable Product in the U.S.

**1.13** “**BMS Claims**” has the meaning set forth in Section 15.1.

- 1.14 “**BMS Indemnitees**” has the meaning set forth in Section 15.1.
- 1.15 “**BMS Patent**” means any Patent that claims a Sole Invention owned by BMS.
- 1.16 “**BMS Proprietary Target**” has the meaning set forth in Section 13.7(a).
- 1.17 “**Budget**” has the meaning set forth in Section 3.2(a).
- 1.18 “**Business Day**” means a day that is not a Saturday, Sunday or a day on which banking institutions in New York, New York, U.S. and/or Amsterdam, the Netherlands are required by Applicable Law to remain closed.
- 1.19 “**Calendar Quarter**” means the respective periods of three (3) consecutive calendar months ending on March 31, June 30, September 30 and December 31.
- 1.20 “**Calendar Year**” means the one (1) year period beginning on January 1 and ending on December 31.
- 1.21 “**Cardiovascular Diseases**” means [\*\*].
- 1.22 “**Certificate**” means a certificate in the form of **Exhibit G**.
- 1.23 “**Change of Control Transaction**” means, with respect to a Party:

(a) the acquisition by any person (within the meaning of Section 13(d)(3) or 14(d)(2) of the Securities Exchange Act of 1934, as amended) (a “**Specified Person**”) of beneficial ownership (within the meaning of Rule 13d-3 promulgated under the Securities Exchange Act of 1934, as amended) of more than fifty percent (50%) of either (i) the then outstanding shares of common stock of such Party (the “**Outstanding Common Stock**”) or (ii) the combined voting power of the then outstanding voting securities

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of such Party entitled to vote generally in the election of directors of such Party (the “**Outstanding Voting Securities**”); provided, however, that for the purposes of this subsection (a), the following acquisitions of securities of such Party shall not constitute a Change of Control Transaction of such Party: (x) any acquisition by such Party, (y) any acquisition by any employee benefit plan (or related trust) sponsored or maintained by such Party or any corporation controlled (within the meaning of Section 1.4 above) by such Party or (z) any acquisition by any corporation pursuant to a transaction which complies with clauses (i) and (ii) of subsection (b) of this definition;

(b) the consummation of any acquisition, merger or consolidation involving any Third Party (a “**Business Combination Transaction**”), unless immediately following such Business Combination Transaction, (i) the individuals and entities who were the beneficial owners, respectively, of the Outstanding Common Stock and Outstanding Voting Securities immediately prior to such Business Combination Transaction beneficially own, directly or indirectly, fifty percent (50%) or more of, respectively, the then outstanding shares of common stock and the combined voting power of the then outstanding voting securities entitled to vote generally in the election of directors, as the case may be, of the corporation or other entity resulting from such Business Combination Transaction (including a corporation which as a result of such transaction owns the then-outstanding securities of such Party or all or substantially all of such Party’s assets either directly or through one or more subsidiaries) in substantially the same proportions as their ownership, immediately prior to such Business Combination Transaction, of the Outstanding Common Stock and Outstanding Voting Securities, as the case may be and (ii) fifty percent (50%) or more of the members of the board of directors of the corporation resulting from such Business Combination Transaction were members of the Board of Directors of such Party at the time of the execution of the initial agreement, or of the action of the Board of Directors of such Party, providing for such Business Combination Transaction; or

(c) a Party or any of its Affiliates sells or transfers to any Specified Person(s) (other than the other Party or its Affiliates) in one or more related transactions properties or assets representing all or substantially all of such Party’s business or assets to which this Agreement relates at the time of such sale or transfer.

- 1.24 “**Claim**” has the meaning set forth in Section 15.3.
- 1.25 “**Clinical Development Forum**” means a forum established by the JSC that will provide a formal setting for keeping uniQure informed of post Early Development activities being conducted by or on behalf of BMS and for seeking input from uniQure on the post Early Development activities.
- 1.26 “**Clinical Trial**” means any human clinical trial of a Product.
- 1.27 “**CMC**” means chemistry, manufacturing and controls with respect to Therapeutics or Products, including the chemistry, manufacturing and controls section of Regulatory Materials for the Products.
- 1.28 “**Collaboration Target**” means the Target encoding S100A1 (also referred to as the “**S100A1 Collaboration Target**”) and any Target that is designated by BMS in writing as being a Collaboration Target in accordance with Section 3.3 and/or Section 3.4 and thereafter so long as it remains a Collaboration Target in accordance with the terms of this Agreement. The Collaboration Target as of the Signing Date is listed in **Exhibit E**. The Collaboration Targets include the New Targets. For clarity, Collaboration Targets shall not include any Reserved Target until such time as such a Reserved Target is designated as a Collaboration Target in accordance with Section 3.3 and/or Section 3.4 (and thereafter so long as it remains a Collaboration Target in accordance with the terms of this Agreement).
- 1.29 “**Collaborator**” has the meaning set forth in Section 7.1(c).
- 1.30 “**Combination Product**” means a Product that includes a Therapeutic and at least one

additional API (whether co-formulated or co-packaged) that is not a Therapeutic, and other than a device. Pharmaceutical dosage form vehicles, buffers, diluents, adjuvants, excipients and similar inert materials shall not be deemed to be “API”, except in the case where such vehicle, buffer, diluent, adjuvant, excipient or similar inert material is recognized by the FDA as an “active ingredient” in accordance with 21 CFR 210.3(b)(7), as amended.

**1.31 “Commercial Forum”** means a forum established by the JSC that will provide a formal setting for keeping uniQure informed of Commercialization activities in the Major Markets being conducted by or on behalf of BMS and for seeking input from uniQure on the Commercialization of Products.

**1.32 “Commercialize” or “Commercialization”** means the marketing, promotion, sale (and offer for sale or contract to sell), distribution, importation or other commercial exploitation (including pricing and reimbursement activities and activities conducted in preparation for a product launch) for a Product (including Products for Gene Therapy and Non-Gene Therapy Products) in the Territory.

**1.33 “Commercialization Wind-Down Period”** has the meaning set forth in Section 13.7(d).

**1.34 “Commercial Plan”** has the meaning set forth in Section 5.2.

**1.35 “Companion Diagnostic”** means a test or assay that is designed and intended to assess whether a patient will or will not respond favorably to a specific drug or other medical treatment, or a degree to which a patient will respond to a specific drug or other medical treatment, or a test or assay without the use of which a drug or other medical treatment cannot be prescribed under the rules and regulations of a Regulatory Authority.

**1.36 “Confidential Information”** means, with respect to a Party, and subject to Section 12.1, all non-public Information of such Party and its Affiliates that is disclosed to the other Party and its Affiliates under this Agreement, and may include specifications, know-how, trade secrets, technical information, models, business information, inventions, discoveries, methods, procedures, formulae, protocols, techniques, data, and unpublished patent applications, whether disclosed in oral, written, graphic, electronic or any other form.

**1.37 “Control”** means, with respect to any material, Information, or intellectual property right, that a Party and/or any Affiliate(s) of such Party (a) owns such material, Information, or intellectual property right, or (b) has a license to or a right to use such material, Information, or intellectual property right, in each case of (a) or (b), with the ability to grant to the other Party access, a right to use, or a license or sublicense (as applicable) to such material, Information, or intellectual property right on the terms and conditions set forth herein, without (i) violating the terms of any agreement or other arrangement with or obligation to any Third Party in existence as of the time such Party and/or any Affiliate(s) of such Party would first be required hereunder to grant the other Party such access, right to use or (sub)license or (ii) paying any sums of money to any Third Party that assigned or licensed such material, Information or intellectual property right to such first Party and/or any Affiliate(s) of such Party that become payable in connection with the other Party’s exploitation thereof hereunder unless (A) such other Party agrees in writing to pay any such sums, subject to Section 8.5(c), or (B) such sums are payable under any Existing License Agreement.

**1.38 “Controlling Party”** has the meaning set forth in Section 7.8(b).

**1.39 “Cover”, “Covered” or “Covering”** means, with respect to a Target, Target Therapeutic, Therapeutic or Product and a Patent, that the composition of matter, method of manufacture or use of such Target, Target Therapeutic, Therapeutic or Product is claimed by a Valid Claim of such Patent (i.e., in the absence of a license under, or ownership of, such Patent, the manufacture, use or sale of such Target, Target Therapeutic, Therapeutic or Product would infringe such Patent as issued or, in the case of a patent application, evaluating such patent application as if it were issued as a Patent as of the date of such

evaluation).

**1.40 “Declined Target”** means a Target other than an Excluded Target that (a) [\*\*], (b) [\*\*], and (c) [\*\*]. For the avoidance of doubt, a Target that does not meet each and every of the criteria set forth in (a) to (c) shall not be considered a Declined Target.

**1.41 “Develop” or “Development”** means all activities that relate to obtaining, maintaining or expanding Regulatory Approval of a Product and to supporting appropriate usage for such Product, for one or more indications in the Field. This includes: (a) non-clinical research and testing, toxicology and Clinical Trials; and (b) preparation, submission, review, and development of data or information and Regulatory Materials for the purpose of submission to a Governmental Authority to obtain, maintain or expand Regulatory Approval of a Product (including contacts with Regulatory Authorities), and outside counsel regulatory legal services related thereto; *provided, however*, that Development shall exclude Commercialization and manufacturing activities (including manufacturing activities related to Development). For clarity, Development shall include Phase 4 Clinical Trials that are required or requested in writing by a Regulatory Authority as a condition of, or in connection with, obtaining or maintaining Regulatory Approval (whether the Phase 4 Clinical Trial is commenced prior to or after receipt of such Regulatory Approval).

**1.42 “Development Plan”** has the meaning set forth in Section 4.1(b).

**1.43 “Diligent Efforts”** means (a) with respect to BMS’ obligations under this Agreement with respect to the conduct of the Research Program and/or its obligations to Develop or Commercialize a Therapeutic or Product, the carrying out of such obligations or tasks with a level of effort and resources consistent with the commercially reasonable practices normally devoted by BMS for the research, Development, manufacture or Commercialization of a biologic pharmaceutical product owned by it, or to which it has exclusive rights, at a similar stage of research, Development, manufacture or Commercialization and of similar market potential and profit potential, based on conditions then prevailing (such efforts may take into account, without limitation, issues of safety and efficacy, regulatory authority-approved labeling, the competitiveness of alternative products in the marketplace, pricing/reimbursement for the product in a country relative to other markets, the likely timing of the product’s entry into the market, the patent and other proprietary position, the likelihood of regulatory approval and other reasonably relevant scientific, technical and commercial factors), and (b) with respect to uniQure’s obligations under this Agreement with respect to the conduct of the Research Program and/or its obligations to Develop a Therapeutic or Product as outlined in the Research Plan and/or the Development Plan, the carrying out of such obligations or tasks with a level of effort and resources consistent with

the commercially reasonable practices normally devoted by uniQure to the carrying out of such obligations or tasks, based on conditions then prevailing, in each case of (a) or (b), subject to and in accordance with the terms and conditions of this Agreement. Without limiting the foregoing, Diligent Efforts shall require [\*\*], at a minimum, to: (x) [\*\*], (y) [\*\*] and (z) [\*\*], and in the case of [\*\*], consistent with what it does for other [\*\*]. For clarity, it is understood and acknowledged that Diligent Efforts in the [\*\*] may include [\*\*].

**1.44** “**Disclosing Party**” has the meaning set forth in Section 12.1.

**1.45** “**Dispute**” has the meaning set forth in Section 16.1.

**1.46** “**Distinct Indication**” means an indication for the prevention, treatment or control of a disease or condition in humans, which is described in the label for the applicable Product (upon Regulatory Approval for such indication), Regulatory Approval for which requires one or more separate registration Clinical Trials for such label; *provided however*, that each such Distinct Indication shall be separate and distinct from each other Distinct Indication. For purposes of this definition, different types, stages, lines of therapy or patient populations for a particular disease shall not be considered to be “separate and distinct” indications from one another, and are not considered separate Distinct Indications (i.e., different types, stages, lines of therapy or patient populations for a particular disease would be considered part of the same

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Distinct Indication). Accordingly, by way of example, dilated cardiomyopathy and hypertrophic cardiomyopathy would be considered to be “separate and distinct” from one another and each would be considered a separate Distinct Indication. Also, by way of example, the treatment of different types, stages, lines of therapy or patient populations of heart failure would not be considered to be “separate and distinct” indications and would all be included as part of one and the same Distinct Indication.

**1.47** “**Dollar**” or “**\$**” means the lawful currency of the United States.

**1.48** “**Drug Delivery Device**” means a drug delivery device for the administration of a Product.

**1.49** “**Early Development**” means Development up to and including [\*\*].

**1.50** “**ECN**” means a Therapeutic that has been designated as an Early Candidate Nomination (or any equivalent successor identifier) by BMS, such that such Therapeutic has been shown to meet the internal standards and criteria established by BMS to qualify the Therapeutic for full pre-clinical development, which standards and criteria are consistent with those customarily used by BMS for its other drug development projects.

**1.51** “**Effective Date**” means the date upon which the Parties have closed the transaction, after all conditions to closing, as set forth in Article 18, have been met.

**1.52** “**EMA**” means the European Medicines Agency and any successor agency thereto.

**1.53** “**Enforce**” or “**Enforcement**” has the meaning set forth in Section 9.5(b).

**1.54** “**Enforcing Party**” has the meaning set forth in Section 9.5(b).

**1.55** “**EU**” means the European Union, as its membership may be constituted from time to time, and any successor thereto, and which, as of the Signing Date, consists of Austria, Belgium, Bulgaria, Croatia, Czech Republic, Denmark, Estonia, Finland, France, Germany, Greece, Hungary, Ireland, Italy, Latvia, Lithuania, Luxembourg, Malta, The Netherlands, Poland, Portugal, Romania, Slovakia, Slovenia, Spain, Sweden and the United Kingdom, and that certain portion of Cyprus included in such organization.

**1.56** “**Excluded Target**” means any Target that is designated by uniQure in writing as being an Excluded Target in accordance with Section 3.4 based on such Target being, at the time of such designation: (i) subject to active discussions between [\*\*] or (ii) subject to [\*\*] under an agreement between [\*\*] with respect to such Target or (iii) subject to an [\*\*] directed against such Target. For the purpose of this Section 1.56, “active discussion” shall mean that [\*\*]. An [\*\*] shall not be included in the [\*\*], the [\*\*] and the licenses and rights granted to BMS hereunder, but shall be [\*\*].

**1.57** “**Executive Officer**” means, in the case of BMS, any senior executive who, depending on the relevant subject matter at issue, reports directly to the Chief Scientific Officer, Chief Financial Officer or Chief Executive Officer of BMS and, in the case of uniQure, and depending on the relevant subject matter at issue, the Chief Scientific Officer, Chief Financial Officer or Chief Executive Officer of uniQure.

**1.58** “**Existing License Agreements**” means the agreements set forth on **Exhibit C**.

**1.59** “**Existing Third Party Licensor**” means a Third Party licensor that is a party to an Existing License Agreement.

**1.60** “**Expert**” means a mutually acceptable, disinterested, conflict-of-interest-free individual not affiliated with either Party or its Affiliates who, with respect to a dispute concerning a financial, commercial, scientific or regulatory matter, possesses appropriate expertise to resolve such dispute. The Expert (or any of

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the Expert’s current or former employers) shall not be or have been at any time an Affiliate, employee, consultant (during the previous five (5) years prior to his/her appointment), officer or director of either Party or any of its Affiliates.

**1.61** “**FDA**” means the United States Food and Drug Administration and any successor agency thereto.

**1.62** “**FD&C Act**” means the United States Federal Food, Drug and Cosmetic Act, as amended.

- 1.63** “**Field**” means all indications and uses, including all human disease indications and uses and diagnostic uses.
- 1.64** “**Financial Representative**” has the meaning set forth in Section 2.3.
- 1.65** “**Financial Transparency Laws**” has the meaning set forth in Section 17.17.
- 1.66** “**First Commercial Sale**” means, with respect to a Product and country, the first sale to a non-Related Party of such Product in such country after Regulatory Approval has been obtained in such country.
- 1.67** “**FTE**” means the equivalent of the work of one appropriately qualified individual working on a full-time basis in performing work in support of the Research Program for a twelve (12) month period (consisting of at least a total of one thousand seven hundred (1,700) hours per year of dedicated effort). No additional payment shall be made with respect to any person who works more than one thousand seven hundred (1,700) hours per year; no person shall count as more than one (1) FTE for any year; and any person who devotes less than one thousand seven hundred (1,700) hours per year shall be treated as an FTE on a pro-rata basis, based upon the actual number of hours worked by such person on the Research Program per year, divided by one thousand seven hundred (1,700). FTE efforts shall not include the work of general corporate or administrative personnel.
- 1.68** “**FTE Rate**” means the yearly rate at which BMS will fund FTEs of uniQure and its Affiliates during the Research Term, which rate is specified in Section 3.5(a).
- 1.69** “**FTE Threshold**” means an overall number of [\*\*] incurred by uniQure or its Affiliates in providing the transfer of uniQure Know-How.
- 1.70** “**GAAP**” means generally accepted accounting principles of the U.S. consistently applied.
- 1.71** “**Gene Therapy**” means the introduction and expression of genetic material in cells of a human in order to cure a disease or to minimize disease symptoms.
- 1.72** “**Generic Product**” means, with respect to a Product, any pharmaceutical product (including a “biogeneric,” “follow-on biologic,” “follow-on biological product,” “follow-on protein product,” “similar biological medicinal product,” or “biosimilar product”) that (a) is (i) in the United States, “similar” or “interchangeable,” with respect to such Product as evaluated by the FDA and falls within the scope of 42 USC 262(i), and (ii) in any country or jurisdiction outside the United States, “similar,” “comparable,” “interchangeable,” “bioequivalent,” or “biosimilar” to such Product, as determined on the basis of any Applicable Law in such country or jurisdiction, including in the EU pharmaceutical products that fall within the definition of a “generic medicinal product” as provided in Article 10, paragraph 2(b) of European Directive 2001/83/EC, or if there is no such Applicable Law, as determined by mutual agreement of the Parties taking into consideration 42 USC 262(i) and Article 10, paragraph 2(b) of European Directive 2001/83/EC, and if the Parties cannot mutually agree, such determination shall be made pursuant to Section 8.5(d), and (b) is not an Authorized Generic Version of such Product; where “**Authorized Generic Version**”

means any pharmaceutical product that (A) is sold under the Regulatory Approval filed by BMS, an Affiliate of BMS or Sublicensee for such Product and (B) has a National Drug Code (or foreign equivalent) (“**NDC**”) number that differs from the NDC number for such Product (other than on a temporary basis as may be necessary to launch such Product in the applicable market).

- 1.73** “**Governmental Authority**” means any supranational, federal, state, local, municipal or other governmental authority of any nature (including any governmental division, subdivision, department, agency, bureau, branch, office, commission, council, court, tribunal or other entity).
- 1.74** “**GMP**” means current Good Manufacturing Practices as specified in the United States Code of Federal Regulations, MHLW regulations, ICH Guideline Q7A, or equivalent laws, rules, or regulations of an applicable Regulatory Authority at the time of manufacture.
- 1.75** “**HSR Act**” means the Hart-Scott-Rodino Antitrust Improvements Act of 1976, as amended, and the rules and regulations of the U.S. Federal Trade Commission thereunder.
- 1.76** [*intentionally left blank*]
- 1.77** “**ICH**” means International Conference on Harmonization of Technical Requirements for Registration of Pharmaceuticals for Human Use.
- 1.78** “**IFRS**” means the International Financial Reporting Standards as issued by the International Accounting Standards Board.
- 1.79** “**IND**” means (a) an Investigational New Drug Application as defined in the FD&C Act and applicable regulations promulgated thereunder by the FDA, or (b) the equivalent application or notification to the applicable Regulatory Authority in any other regulatory jurisdiction, the filing of which is necessary to initiate or conduct clinical testing of a pharmaceutical product in humans in such jurisdiction.
- 1.80** “**Indemnified Party**” has the meaning set forth in Section 15.3.
- 1.81** “**Indemnifying Party**” has the meaning set forth in Section 15.3.
- 1.82** “**Information**” means any data, results, and information of any type whatsoever, in any tangible or intangible form, including know-how, trade secrets, practices, techniques, methods, processes, inventions, developments, specifications, formulations, formulae, materials or compositions of matter of any type or kind (patentable or otherwise), software, algorithms, marketing reports, expertise, stability, technology, test data including pharmacological, biological, chemical, biochemical, toxicological, and clinical test data, analytical and quality control data, stability data, studies and procedures.
- 1.83** “**Infringement**” has the meaning set forth in Section 9.5(a).
- 1.84** “**Infringement Action**” has the meaning set forth in Section 9.5(b).

- 1.85 “**Initial Commercial Plan**” has the meaning set forth in Section 5.2.
- 1.86 “**Initial Development Plan**” has the meaning set forth in Section 4.1(b).
- 1.87 “**Initial Research Term**” has the meaning set forth in Section 3.1(e).
- 1.88 “**Insolvency Event**” has the meaning set forth in Section 13.5.
- 1.89 “**Inventor Compensation**” has the meaning set forth in Section 8.17.

- 1.90 “**JNDA**” means a new drug application, for which Regulatory Approval by the MHLW is required to market a Product in Japan.
- 1.91 “**JNDA Approval**” means, with respect to a Product, receipt of Regulatory Approval of a JNDA by the MHLW for such Product in Japan.
- 1.92 “**JNDA Filing**” means the acceptance by the PMDA of the filing of a JNDA with the PMDA for the applicable Product in Japan.
- 1.93 “**Joint CMC Working Group**” means a working group established by the JSC that will manage CMC activities for Therapeutics and Products.
- 1.94 “**Joint Discovery Working Group**” means a working group established by the JSC that will manage research discovery activities directed to identifying and validating Collaboration Targets and identifying and discovering Therapeutics.
- 1.95 “**Joint Early Development Working Group**” means a working group established by the JSC that will provide input on and manage the Development activities for each Therapeutic and Product (in particular each ECN) through a proof-of-concept (POC) Clinical Trial for each Therapeutic and Product (in particular each ECN).
- 1.96 “**Joint Inventions**” has the meaning set forth in Section 9.1(b).
- 1.97 “**Joint Patent**” means a Patent that claims a Joint Invention.
- 1.98 “**Joint Regulatory Working Group**” means a working group established by the JSC that will provide Gene Therapy regulatory guidance regarding the nature and frequency of interactions with Regulatory Authorities as well as provide guidance and support on which issues should be raised and addressed with Regulatory Authorities in connection with the Development of Therapeutics and Products, will assist in the preparation and review of Regulatory Materials, and will monitor the submission of Regulatory Materials for each Therapeutic and Product (in particular each ECN) through the First Commercial Sale of each such Therapeutic and Product.
- 1.99 “**JSC**” has the meaning set forth in Section 2.1(a).
- 1.100 “**Know-How Transfer Purposes**” has the meaning set forth in Section 3.13.
- 1.101 “**Lead S100A1 Back-up Therapeutics**” means any Back-up Therapeutic of the Lead S100A1 Therapeutics.
- 1.102 “**Lead S100A1 Therapeutics**” means the following Therapeutics existing as of the Signing Date: (a) the Therapeutic for the Target [\*\*], and (b) the Therapeutic for the [\*\*].
- 1.103 “**Liens**” means any lien, pledge, encumbrance, mortgage, security interest, purchase option, call or similar right, conditional and installment sale agreements, charges or claims of any kind (excluding any license or other rights granted to Third Parties under any of the uniQure Technology that do not conflict with or otherwise limit the rights granted to BMS under this Agreement).
- 1.104 “**MAA**” means a marketing authorization application, for which Regulatory Approval by the European Commission is required to market a Product in the EU or, to the extent the centralized EMA filing procedure is not used, a marketing authorization application, for which Regulatory Approval by the Regulatory Authority in any Major European Country is required to market a Product in such Major European Country.

- 1.105 “**MAA Approval**” means, with respect to a Product, receipt of Regulatory Approval of an MAA by the European Commission for such Product in the EU or, to the extent the centralized EMA filing procedure is not used, receipt of the first Regulatory Approval of an MAA by the Regulatory Authority in any Major European Countries.
- 1.106 “**MAA Filing**” means validation by the EMA of the filing of an MAA with the EMA for the applicable Product under the centralized European procedure, as demonstrated by the start of the procedure under the timetable adopted by the Committee for Medicinal Products for Human Use (CHMP), or, to the extent the centralized EMA filing procedure is not used, the first acceptance of the filing of an MAA with the applicable Regulatory Authority in any Major European Countries.
- 1.107 “**Major European Countries**” means [\*\*] and the [\*\*].
- 1.108 “**Major Markets**” means the [\*\*] and the Major European Countries, and “**Major Market**” means any of the [\*\*] and any Major European Country.

**1.109 “Manufacturing Cost-Based Component of Supply Price”** has the meaning set forth in **Exhibit J** and the definition will be included in the Supply Agreement.

**1.110 “MCOs”** means pharmacies, managed health care organizations, group purchasing organizations, large employers, long-term care organizations, formularies, insurers, government agencies and programs (e.g., Medicare and the VHA and other federal, state and local agencies), or similar organizations.

**1.111 “MHLW”** means the Japanese Ministry of Health, Labour and Welfare and any successor agency thereto.

**1.112 “Net Sales”** means the gross amounts invoiced in arms-length transactions from or on account of the sale, leasing, distribution or other transfer of Products (including Products for Gene Therapy and Non-Gene Therapy Products) by any Related Party to a non-Related Party, less the sum of the following:

- (a) credits or allowances for price adjustments, recalls, claims, damaged goods, rejections or returns of Products previously sold to such non-Related Party (including Products returned in connection with recalls or withdrawals);
- (b) taxes on the sale of Products to such non-Related Party (including import taxes, export taxes, excise taxes (including annual fees due under Section 9008 of the United States Patient Protection and Affordable Care Act of 2010 (Pub. L. No. 111-48)), sales taxes, value-added taxes, consumption taxes, duties or other taxes levied on or imposed with respect to such sale (excluding income or net profit taxes or franchise taxes of any kind)), to the extent not reimbursed by such non-Related Party;
- (c) insurance, customs charges, freight, shipping and other transportation costs actually incurred in shipping Products to such non-Related Party, to the extent not reimbursed by such non-Related Party;
- (d) discounts (including trade, quantity and cash discounts), cash and non-cash coupons, retroactive price reductions, and charge-back payments and rebates granted to any non-Related Party (including to governmental entities or agencies, purchasers, reimbursers, customers, distributors, wholesalers, and group purchasing and MCOs (and other similar entities and institutions)); and
- (e) rebates (or their equivalent), administrative fees, chargebacks and retroactive price adjustments and any other similar allowances granted to non-Related Parties (including to Governmental Authorities, purchasers, reimbursers, customers, distributors, wholesalers, and MCOs (and other similar entities and institutions)) which effectively reduce the selling price or gross sales of the Product.

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No deduction shall be made for any item of cost incurred by any Related Party in Developing or Commercializing Products except as permitted pursuant to clauses (a) to (e) of the foregoing sentence, *provided however*, that Products transferred to non-Related Parties in connection with clinical and non-clinical research and trials, Product samples, compassionate sales or use, or an indigent program or for similar bona fide business purposes in accordance with local Applicable Law shall give rise to Net Sales only to the extent that any Related Party invoices or receives amounts therefor. Such amounts shall be determined consistent with customary practices and in accordance with GAAP.

No amount for which deduction is permitted pursuant to this Section 1.112 shall be deducted more than once. Products shall be considered “sold” when invoiced to the non-Related Party.

In no event shall any Related Party have a right to apply any discounts or deductions on the Product resulting from the Related Party entering into “package deals” whereby the Related Party sells more than one product (in addition to the Product) to a customer and offers “package deal discounts”.

Sale or transfer of Products between any of the Related Parties shall not result in any Net Sales, with Net Sales to be based only on any subsequent sales or dispositions by a Related Party to a non-Related Party, unless such Related Party is an end-user of such Products. To the extent that any Related Party receives consideration other than or in addition to cash upon the sale or disposition of Products to a non-Related Party, Net Sales shall include the fair market value of such additional consideration for such sale or disposition. For clarity, (i) Net Sales shall not include amounts or other consideration received by a Related Party from a non-Related Party in consideration of the grant of a (sub)license or co-promotion or distribution right to such non-Related Party, (ii) sales to a Third Party wholesaler or distributor, group purchasing organization, pharmacy benefit manager, or retail chain customer shall be considered sales to a non-Related Party and not to a Sublicensee; (iii) Net Sales by a Related Party to a non-Related Party consignee are not recognized as Net Sales by such Related Party until the non-Related Party consignee sells the Product to any Third Party and (iv) Net Sales Compensation shall only be paid one time on each unit of Product.

Net Sales of any Combination Product for the purpose of calculating milestones or Net Sales Compensation due under this Agreement shall be determined on a country-by-country basis for a given accounting period as follows: first, the Related Party(ies) shall determine the actual Net Sales of such Combination Product (using the above provisions), and then:

(1) Such Net Sales amount for the Combination Product shall be multiplied by the fraction  $A/(A+B)$ , where A is the net selling price in such country of a Product containing only the applicable Therapeutic, if sold separately for the same dosage as contained in the Combination Product, and B is the net selling price in such country of any other API in the combination, if sold separately for the same dosage as contained in the Combination Product. All net selling prices of the elements of such end-user product or service shall be calculated as the average net selling price of the said elements during the applicable accounting period for which the Net Sales are being calculated; or

(2) In the event that, in any country, no separate sale of either such above-designated Product (containing only the applicable Therapeutic and no other API) or any one or more of the other APIs included in such Product are made during the accounting period in which the sale was made, or if the net selling price for an API cannot be determined for an accounting period, the allocation methodology in estimating fair market value of the Net Sales allocable to the Product in each such country shall be determined by mutual agreement reached in good faith by the Parties prior to the end of the accounting period in question based on an equitable method of determining the same that takes into account, on a country-by-country basis, all relevant factors (including variations in potency, the relative contribution of each API in the combination, and relative value to the end user of each API). BMS shall make a written proposal to uniQure, for its review of such allocation methodology in estimating fair market value, reasonably supported with appropriate documentation. If the Parties are unable to reach agreement on such matter, the provisions of Article 16 shall apply.



1.113 “**Net Sales Compensation**” has the meaning set forth in Section 8.5(a)(ii).

1.114 “**New Target**” has the meaning set forth in Section 3.3(a)(i).

1.115 “**Non-Controlling Party**” has the meaning set forth in Section 7.8(b).

1.116 “**Non-Gene Therapy Products**” has the meaning set forth in Section 6.2.

1.117 “**Non-Gene Therapy Therapeutics**” has the meaning set forth in Section 6.2.

1.118 “**Other Joint Patents**” has the meaning set forth in Section 9.4(a).

1.119 “**Other uniQure Patents**” has the meaning set forth in Section 9.4(c).

1.120 “**Patents**” means (a) all patents and patent applications, including provisional patent applications, (b) all patent applications filed either from such patents, patent applications or provisional applications or from an application claiming priority from any of these, including divisionals, continuations, continuations-in-part, converted provisionals, and continued prosecution applications, (c) any and all patents that have issued or in the future issue from the foregoing patent applications in (a) and (b), including utility models, petty patents and design patents and certificates of invention, and (d) any and all extensions or restorations by existing or future extension or restoration mechanisms, including adjustments, revalidations, reissues, re-examinations and extensions (including any supplementary protection certificates and the like) of the foregoing patents or patent applications in (a), (b) and (c).

1.121 “**Patent Challenge**” has the meaning set forth in Section 9.9(a).

1.122 “**Patent Contact**” has the meaning set forth in Section 9.11.

1.123 “**Patent Firm**” has the meaning set forth in Section 9.2(a).

1.124 “**Patent Prosecution Costs**” means the direct out-of-pocket costs and expenses (including the reasonable fees and expenses incurred to outside counsel and other Third Parties, including filing, prosecution and maintenance fees incurred to Patent offices and other Governmental Authorities) recorded as an expense by a Party or any of its Affiliates (in accordance with GAAP for BMS and IFRS for uniQure and its customary accounting practices) after the Effective Date and during the Term and pursuant to this Agreement, in connection with the preparation, filing, prosecution, maintenance and extension of Patents, including costs of Patent interference, appeal, opposition, reissue, reexamination, revocation, petitions or other administrative proceedings with respect to Patents and filing and registration fees.

1.125 “**PMDA**” means the Pharmaceuticals Medical Devices Agency in Japan and any successor agency thereto.

1.126 “**Person**” means any individual, firm, corporation, partnership, limited liability company, trust, business trust, joint venture company, Governmental Authority, association or other entity.

1.127 “**Phase 1 Clinical Trial**” means a Clinical Trial of a Product on sufficient numbers of normal volunteers and/or patients that is designed to establish that such Product is safe for its intended use and to support its continued testing in Phase 2 Clinical Trials.

1.128 “**Phase 2 Clinical Trial**” means a Clinical Trial of a Product that utilizes the pharmacokinetic and pharmacodynamic information obtained from one or more Phase 1 Clinical Trial(s) that is designed to provide a preliminary determination of safety and efficacy of such Product in the target patient population over a range of doses and dose regimens.

1.129 “**Phase 3 Clinical Trial**” means a Clinical Trial of a Product on sufficient numbers of

patients that is designed to establish that such Product is safe and efficacious for its intended use, and to define warnings, precautions and adverse reactions that are associated with such Product in the dosage range to be prescribed, and to support Regulatory Approval of such Product or label expansion of such Product.

1.130 “**Phase 4 Clinical Trial**” means a Clinical Trial of a Product that (a) is not required for receipt of initial Regulatory Approval of such Product for a particular country or jurisdiction in the Territory but which may be useful in providing additional drug profile data in support of such Regulatory Approval (whether the trial is commenced prior to or after receipt of such Regulatory Approval), or (b) is required, requested or advised by a Regulatory Authority as a condition of, or in connection with, obtaining or maintaining Regulatory Approval (whether the trial is commenced prior to or after receipt of such Regulatory Approval). Phase 4 Clinical Trials may include trials or studies conducted in support of pricing and reimbursement approvals, epidemiological studies, modeling and pharmacoeconomic studies, post-marketing surveillance studies, investigator sponsored Clinical Trials and health economics studies.

1.131 “**Prior CDA**” means the Confidentiality Agreement entered into by BMS and uniQure N.V. effective September 18, 2014, as amended by the first amendment dated January 26, 2015 and the second amendment dated February 24, 2015.

1.132 “**Product**” means any pharmaceutical product containing a Therapeutic (alone or with other APIs), in all forms, presentations, formulations, methods of administration and dosage forms.

1.133 “**Product Marks**” has the meaning set forth in Section 10.1.

1.134 “**Product Liability Losses**” has the meaning set forth in Section 15.4.

1.135 “**Product Specific Infringement Action**” has the meaning set forth in Section 9.5(b).

1.136 “**Product Specific Patents**” means any [\*\*] (including all claims and the entire scope of claims therein) which (a) are not [\*\*] and [\*\*] or thereafter during the [\*\*] or any of its Affiliates and which (b) contain one or more [\*\*], [\*\*] of either (i) one or more particular [\*\*] (where such claim specifically recites the [\*\*]) or (ii) one or more [\*\*] as well as one or more [\*\*] or products that are not [\*\*] (where such claim specifically recites the [\*\*]). For the avoidance of doubt, Product Specific Patents shall not contain, (x) [\*\*], and (y) [\*\*] (including all [\*\*]) [\*\*] or thereafter during the [\*\*] or any of its Affiliates that constitutes [\*\*]. As of the Signing Date, the Product Specific Patents consist of the [\*\*].

1.137 “**Prosecute**” or “**Prosecution**” has the meaning set forth in Section 9.2(a).

1.138 “**Prosecuting Party**” has the meaning set forth in Section 9.2(a).

1.139 “**Publication**” has the meaning set forth in Section 12.4.

1.140 “**Raw Materials**” means the Transgene, vector and promoter that are contained in a Therapeutic and any starting materials, intermediates, cell lines, virus stocks and media used in the generation of such Transgene, vector and promoter or otherwise in the manufacture of a Therapeutic, including the API form of a Therapeutic.

1.141 “**Receiving Party**” has the meaning set forth in Section 12.1.

1.142 “[\*\*]” has the meaning set forth in Section 8.5(c)(vi).

1.143 “**Regulatory Approval**” means with respect to a country, extra-national territory, province, state, or other regulatory jurisdiction, any and all approvals, licenses, registrations or authorizations of any Regulatory Authority necessary in order to Commercialize a Product in such country, state, province, or

some or all of such extra-national territory or regulatory jurisdiction, but which shall exclude any pricing and reimbursement approvals.

1.144 “**Regulatory Authority**” means, with respect to a particular country, extra-national territory, province, state, or other regulatory jurisdiction, any applicable Governmental Authority involved in granting Regulatory Approval or, to the extent required for such country, extra-national territory, province, state, or other or regulatory jurisdiction, pricing and reimbursement approval of a Product in such country, state, province, or some or all of such extra-national territory or regulatory jurisdiction, including the FDA, the EMA, the European Commission and the MHLW, and in each case including any successor thereto.

1.145 “**Regulatory Materials**” means regulatory applications, submissions, dossiers, notifications, registrations, Regulatory Approvals and other filings made to or with, or other approvals granted by, a Regulatory Authority that are necessary or reasonably desirable in order to Develop, manufacture or Commercialize a Product in a particular country or regulatory jurisdiction. Regulatory Materials include INDs, BLAs, MAAs and JNDAs.

1.146 “**Related Party**” shall mean BMS and its Affiliates and their respective Sublicensees and such Sublicensees’ Affiliates of one or more Therapeutics or Products. For clarity, Related Party shall not include any wholesalers or distributors or the like unless such entity is an Affiliate of BMS.

1.147 “**Replaced Target**” has the meaning set forth in Section 3.3(d)(i).

1.148 “**Replacement Target**” has the meaning set forth in Section 3.3(c)(ii).

1.149 “**Research Plan**” has the meaning set forth in Section 3.2(a).

1.150 “**Research Program**” has the meaning set forth in Section 3.1(a).

1.151 “**Research Program Costs**” has the meaning set forth in Section 3.5(c).

1.152 “**Research Term**” has the meaning set forth in Section 3.1(e).

1.153 “**Research Year**” means each twelve (12) month period during the Research Term, with the first Research Year beginning on the Effective Date.

1.154 “**Reserved Target**” has the meaning set forth in Section 3.3(b)(i).

1.155 “**S100A1**” means the cardiomyocyte protein S100A1.

1.156 “**Safety Reason**” means it is BMS’ or any BMS Wholly Owned Affiliate’s reasonable belief that based upon an analysis of the existing information at any time, the medical risk/benefit of a Therapeutic or Product is sufficiently unfavorable as to be incompatible with the welfare of patients to Develop or Commercialize or to continue to Develop or Commercialize it, which Safety Reason (including a summary of the data supporting such reason) shall be disclosed to uniQure.

1.157 “**SEC**” means the U.S. Securities and Exchange Commission and any successor agency thereto.

1.158 “**Second Designation Date**” means the later of (a) the [\*\*] year anniversary of the Effective Date, and (b) the [\*\*].

1.159 “[\*\*] **New Targets**” has the meaning set forth in Section 3.3(a)(iii).

1.160 “**Signing Date**” has the meaning set forth in the first paragraph of this Agreement.

1.161 “**Sole Inventions**” has the meaning set forth in Section 9.1(a).

1.162 “**Sublicensee**” means any Third Party (a) granted a sublicense by BMS under Section 7.2 hereof to any or all of the rights licensed to BMS hereunder, or (b) to which BMS otherwise grants the right to promote and sell Products in one or more countries of the Territory (and any subsequent Sublicensee of such Sublicensee (i.e., in multiple tiers)), but excluding any Third Party wholesaler, distributor, group purchasing organization, pharmacy benefit manager, or retail chain customer (or similar purchaser) even if such Third Party purchaser is granted a right or license to sell a Product.

1.163 “**Supply Agreement**” has the meaning set forth in Section 6.2.

1.164 “**Target**” means any Transgene.

1.165 “**Target Designation Fee**” means the applicable fee to be paid by BMS for the designation of a Target as a New Target in accordance with Section 3.3(a).

1.166 “**Target Reviewer**” has the meaning set forth in Section 3.4(a).

1.167 “**Target Therapeutic**” means a construct that contains a Target, a promoter and a vector.

1.168 “**Term**” has the meaning set forth in Section 13.1.

1.169 “**Terminated Products**” has the meaning set forth in Section 13.7(a).

1.170 “**Terminated Target**” has the meaning set forth in Section 13.7(a).

1.171 “**Terminated Therapeutics**” has the meaning set forth in Section 13.7(a).

1.172 “**Termination Notice**” has the meaning set forth in Section 13.3(a).

1.173 “**Territory**” means all countries of the world.

1.174 “**TC Term**” has the meaning set forth in Section 8.5(e).

1.175 “**Therapeutic**” means (a) any [\*\*] discovered, owned or Controlled by or for [\*\*] as part of the performance of the [\*\*], (b) any [\*\*] discovered by or for [\*\*] (i.e., whether or not as part of the performance of the [\*\*]) as of the Effective Date or thereafter during the Term, (c) any [\*\*] or any [\*\*] with a [\*\*] that is generically or specifically claimed by a [\*\*], (d) any [\*\*] discovered by [\*\*] as part of the performance of the [\*\*], and (e) any [\*\*] which [\*\*] manufacture, approved use or sale thereof would infringe a [\*\*] but for [\*\*]. Therapeutics include, but are not limited to, the [\*\*].

1.176 “**Third Designation Date**” means the date that is the later of (a) the [\*\*] anniversary of the Effective Date (if the Research Term has been extended to be at least [\*\*] in duration) or, as the case may be, the [\*\*] anniversary of the Effective Date (if the Research Term has not been extended beyond the Initial Research Term), and (b) the date of the [\*\*].

1.177 “**Third Party**” means any Person other than uniQure or BMS or an Affiliate of either of uniQure or BMS.

1.178 “**Third Party Costs**” means out-of-pocket costs and expenses to be incurred by uniQure or its Affiliates (i.e., payments to be made by uniQure or its Affiliates to Third Parties) in conducting the

activities assigned to uniQure or its Affiliates or such Third Party pursuant to the then-current Research Plan and in accordance with the Budget for such activities as agreed to by the JSC and set forth in the Research Plan. Third Party Costs may include, for example, costs for animals to be used specifically in the Research Program or costs for studies performed by outside (sub)contractors, but shall not include costs for routine laboratory supplies and applicable overhead costs of uniQure or its Affiliates. For the avoidance of doubt, Third Party Costs exclude all license fees, upfront payments, milestones and royalties paid to Third Parties, including but not limited to any amounts paid by uniQure to Existing Third Party Licensors under Existing License Agreements.

1.179 “**Third Party Licenses**” has the meaning set forth in Section 7.7(a).

1.180 “[\*\*] **New Targets**” has the meaning set forth in Section 3.3(a)(iv).

1.181 “**Title 11**” has the meaning set forth in Section 17.3(a).

1.182 “**Total Compensation**” has the meaning set forth in Section 8.5(a).

1.183 “**Transaction Agreements**” has the meaning set forth in Section 8.2.

1.184 “**Transgene**” means (a) a segment of DNA containing a gene sequence that has been isolated and is intended to be introduced into a human cell to express a particular protein of interest, or (b) an oligonucleotide sequence encoding a small RNA sequence which is involved in the knock-down (i.e.

the suppression, reduction or inhibition of protein translation) of a specific human gene or micro RNA.

**1.185 “uniQure Claims”** has the meaning set forth in Section 15.2.

**1.186 “uniQure Indemnitees”** has the meaning set forth in Section 15.2.

**1.187 “uniQure Know-How”** means all Information Controlled as of the Effective Date or thereafter during the Term by [\*\*] that encompass or relate to [\*\*] or that is necessary or reasonably useful for the [\*\*] or the [\*\*]. uniQure Know-How includes (a) [\*\*] and (b) to the extent Controlled as of the Effective Date or thereafter during the Term by [\*\*], (i) [\*\*], (ii) all [\*\*] and other methods of [\*\*] (including their [\*\*]), and (iii) all [\*\*]. uniQure Know-How shall exclude rights under any [\*\*]. For clarity, subject to and to the extent as provided in Section 17.8, the use of “Affiliate” in this definition shall exclude any Third Party that becomes an Affiliate due to [\*\*].

**1.188 “uniQure Manufacturing Know-How”** means all uniQure Know-How that is [\*\*], including Information with respect to the [\*\*] (or any intermediate of any of the foregoing), including with respect to [\*\*].

**1.189 “uniQure Manufacturing Patents”** means all [\*\*] that are [\*\*] as of the Effective Date or thereafter during the Term by [\*\*] and that (a) contain [\*\*], and (b) do not contain [\*\*]. As of the Signing Date, the uniQure Manufacturing Patents are the [\*\*].

**1.190 uniQure Manufacturing Technology”** means the uniQure Manufacturing Patents and the uniQure Manufacturing Know-How.

**1.191 “uniQure Materials”** means all tangible materials, including Raw Materials, in the possession and Control of uniQure or any of its Affiliates as of the Effective Date or thereafter during the Research Term that are necessary or reasonably useful for (a) the discovery, identification or validation of Collaboration Targets or (b) the discovery, evaluation, Development, manufacture or Commercialization of Therapeutics or Products.

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**1.192 “uniQure Patents”** means all [\*\*] that are [\*\*] as of the Effective Date or thereafter during the Term by [\*\*] and that [\*\*] or that are necessary or reasonably useful for the [\*\*] or the [\*\*]. uniQure Patents include the [\*\*] that are considered uniQure Patents pursuant to [\*\*], and [\*\*]. For clarity, subject to and to the extent as provided in [\*\*], the use of “Affiliate” in this definition shall exclude any Third Party that becomes an Affiliate due to [\*\*]. As of the Signing Date, the uniQure Patents include the [\*\*].

**1.193 “uniQure Platform Technology”** means all [\*\*] as of the Effective Date or thereafter during the Term by [\*\*] that have [\*\*] to the research and development of [\*\*], their manufacturing as well as administration to [\*\*].

**1.194 “uniQure Platform Technology Improvements”** means any [\*\*] to the [\*\*] which is [\*\*].

**1.195 “uniQure Technology”** means the uniQure Patents, uniQure Know-How and uniQure Materials.

**1.196 “U.S.” or “United States”** means the United States of America **and its** territories, districts and possessions.

**1.197 “Valid Claim”** means either (a) a claim of an issued and unexpired Patent which has not been held permanently revoked, unenforceable or invalid by a decision of a court or other Governmental Authority of competent jurisdiction, unappealable or unappealed within the time allowed for appeal and that is not admitted to be invalid or unenforceable through reissue, disclaimer or otherwise (i.e., only to the extent the subject matter is disclaimed or is sought to be deleted or amended through reissue), or (b) a claim of a pending Patent application that has not been abandoned, finally rejected or expired without the possibility of appeal or refiling; *provided however*, that Valid Claim shall exclude (x) any such claim of a pending Patent application that has not been granted within [\*\*] following the earliest priority filing date for such application and (y) any such claim of a pending Patent application that does not have a reasonable bona fide basis for patentability (such reasonable bona fide basis to be determined by arbitration pursuant to Section 16.2 (with the/each arbitrator being a patent attorney having appropriate expertise in patent law) in the event that the Parties disagree as to whether there is a reasonable bona fide basis for patentability for such a claim), in either case of (x) or (y) unless and until such claim is granted and in such event, such claim shall retroactively be deemed to have been a Valid Claim.

**1.198 “Variant”** with respect to a Target means a [\*\*] containing [\*\*], that has or is intended to have [\*\*] substantially similar to, or superior to, [\*\*].

**1.199 “Wholly Owned Affiliate”** means, with respect to a particular Person, another Person that (i) directly or indirectly owns one hundred percent (100%) of the voting stock of such particular Person, (ii) the voting stock of such other Person is directly or indirectly owned one hundred percent (100%) by such particular Person, or (iii) the voting stock of such other Person is directly or indirectly owned one hundred percent (100%) by a Person that also owns directly or indirectly one hundred percent (100%) of the voting stock of such Particular person.

## 2. GOVERNANCE

### 2.1 Joint Steering Committee.

(a) **Establishment of JSC.** The Parties will establish a joint steering committee with the roles set forth in Section 2.1(c) (the “JSC”) no later than thirty (30) days after the Effective Date. Each Party will initially appoint three (3) representatives to the JSC. The initial members of the JSC are identified

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in **Exhibit F**. The JSC may change its size from time to time by mutual consent of its members; *provided however*, that the JSC will consist at all times of an equal number of representatives of each of uniQure and BMS. The JSC membership and procedures are further described in this Section 2.1. Each Party may at any time appoint different JSC representatives by written notice to the other Party.

(b) **Membership of JSC.** Each of uniQure and BMS will designate representatives with appropriate expertise to serve as members of the JSC. Each of uniQure and BMS will select from their representatives a co-chairperson for the JSC, and each Party may change its designated co-chairperson from time to time upon written notice to the other Party.

(c) **Role of JSC.** The JSC will be responsible for (i) oversight for all aspects of the collaboration of uniQure and BMS under this Agreement, (ii) oversight for and the overall management (through the Joint Discovery Working Group) of the Research Program, and for approving changes and updates to the Research Plan and the Budget, (iii) oversight for and the overall management (through the Joint Early Development Working Group) of the Early Development activities for Therapeutics and Products, (iv) seeking input from uniQure on the Development by BMS of Therapeutics and Products and monitoring the Development progress of each Therapeutic and Product by BMS, (v) oversight for and the overall management (through the Joint CMC Working Group) of CMC activities in connection with the Development of Therapeutics and Products, (vi) monitoring, reviewing and recording of the progress of the Research Program, (vii) setting and monitoring the spending against the Budget for Research Program Costs, as set forth in the Research Plan, (viii) facilitating the prosecution of the Product Specific Patents in accordance with Article 9 below (and for the avoidance of doubt the JSC will have no authority to determine whether a Patent is a Product Specific Patent), (ix) documenting the designation by BMS of Collaboration Targets (including the replacement of a Collaboration Target with a Replacement Target), (x) providing Gene Therapy regulatory guidance regarding the nature and frequency of interactions with Regulatory Authorities and issues to be raised and addressed with Regulatory Authorities in connection with the Development of Therapeutics and Products, (xi) providing assistance in the review of Regulatory Materials, and monitoring the submission of Regulatory Materials for each Therapeutic and Product (in particular each ECN) through the First Commercial Sale of each such Therapeutic and Product, and (xii) reviewing the Commercialization activities of each Therapeutic and Product (in particular each ECN) by BMS and other Related Parties. The JSC shall also serve as an information sharing forum for keeping uniQure informed of Development activities being conducted by BMS and other Related Parties for Therapeutics and Products. As needed, the JSC shall establish subcommittees and working groups (which in each case shall include representation by each Party but it shall be within the discretion of the JSC whether an equal number of representatives from each Party should be on a particular subcommittee or working group) that will report to the JSC to further the objectives of the Research Program and the Development and Commercialization of Therapeutics and Products. Without limiting the foregoing, within thirty (30) days of the Effective Date the JSC shall establish the Joint Discovery Working Group, and within sixty (60) days of the Effective Date, the JSC shall establish the Joint Early Development Working Group, the Joint Regulatory Working Group and the Joint CMC Working Group. Reasonably in advance of the commencement of the first Clinical Trial for a Product, the JSC shall establish the Clinical Development Forum, and reasonably in advance of the commencement of any Commercialization activities, the JSC shall establish the Commercial Forum. The number of members from each Party for the Clinical Development Forum and the Commercial Forum do not need to be equal, and there is no limit on the number of participants from each Party that may attend a meeting of the Clinical Development Forum or of the Commercial Forum.

(d) **Decisions of JSC.** Decisions of the JSC shall be taken during meetings of the JSC pursuant to Section 2.1(f). The JSC shall only have decision making authority on matters falling within the scope of clauses (ii), (iii), (v), (vi) and (vii) of Section 2.1(c). Decisions of the JSC on such matters shall be by consensus and the JSC representatives of a Party shall consider the views of the JSC representatives of the other Party in making decisions. Each Party, through its representatives, shall have one (1) vote in the JSC. If the JSC is unable to reach consensus with respect to any such decision, the disputed matter will be subject

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to the dispute resolution mechanism set forth in Section 2.1(e). BMS shall have the final decision-making authority on matters falling within the scope of the JSC; *provided however*, that BMS may not use its final decision-making authority to (i) require uniQure or any of its Affiliates to violate any Applicable Law or any agreement it may have with any Related Party or Third Party, (ii) amend the terms and conditions of this Agreement, (iii) make any changes in the number of BMS-funded uniQure FTEs except in accordance with Section 3.5(b), (iv) require uniQure or any of its Affiliates to incur any additional efforts and costs (other than routine laboratory supplies up to an amount of [\*\*] per Research Year) in the conduct of the Research Program beyond the efforts and costs specified in the last agreed Budget for the Research Plan or (v) require uniQure or any of its Affiliates to conduct any activities outside the scope of the discovery, research, production, manufacture or non-clinical Development of Collaboration Targets and Therapeutics as set forth in this Agreement and, as the case may be, the Supply Agreement. For clarity and subject to Section 3.5, BMS shall have final decision-making authority with respect to the allocation of FTE effort to be applied for the conduct of work with respect to each of the Collaboration Targets.

(e) **Dispute Resolution.** In the case where the JSC is unable to reach a decision by consensus within fifteen (15) days of a matter for which it has decision making authority first being voted upon by the JSC, the disputed matter shall then be immediately referred to the Alliance Managers for good faith discussion and resolution, and if it is not resolved by the Alliance Managers within ten (10) days of being referred to them, such disputed matter shall be immediately referred to the Executive Officers for each Party in charge of the relevant subject matter at issue for resolution within fifteen (15) days. If, after such efforts, the Parties are unable to resolve such disputed matter, the Executive Officer of BMS (or his or her designee) shall have the final decision making authority to decide the disputed matter, subject to Section 2.1(d).

(f) **JSC Meetings.** The co-chairpersons of the JSC, with assistance and guidance from the Alliance Managers, will be responsible for calling and leading meetings, preparing and circulating an agenda in advance of each meeting and minutes of meeting promptly after each meeting (including a list of any actions or decisions approved by the JSC). The JSC will hold meetings at such times and places as the co-chairpersons may determine; *provided however*, that the co-chairpersons will call a meeting of the JSC promptly upon the reasonable written request of either co-chairperson to convene such a meeting. The JSC will meet at least once every Calendar Quarter unless the Parties agree otherwise. At least two (2) meetings each Calendar Year shall be in person. The location of in-person JSC meetings shall alternate between the headquarters of uniQure and either the headquarters of BMS or a BMS facility in New Jersey, United States (with the BMS co-chairperson selecting the site of each in person meeting to be held at a BMS location), with the first meeting to take place at uniQure in Amsterdam, the Netherlands. The other meetings of the JSC in a Calendar Year need not be in person and may be by telephone or any other method determined by the JSC. Each Party shall use reasonable efforts to cause its representatives to attend the meetings of the JSC and any subcommittee or working group. In addition, each Party may, at its discretion, invite a reasonable number of non-voting employees or officers, and, with the consent of the other Party, consultants or scientific advisors, to attend meetings of the JSC or any subcommittee or working group, or the relevant portion thereof; *provided however*, that the total number of non-members from each Party that may attend a meeting will be decided by the Alliance Managers and that its representatives and any such other employees, officers, consultants or scientific advisors are bound by written obligations of confidentiality that are at least as stringent as those set forth in this Agreement. Each Party shall bear all travel and other expenses of its representatives and other employees, officers, consultants or scientific advisors incurred to attend the meetings of the JSC or any subcommittee or working group.

(g) **Discontinuation of JSC.** The JSC shall continue to exist until the first to occur of (a) the Parties mutually agreeing to disband the JSC, or (b) termination or expiration of this Agreement. Thereafter the JSC shall have no further roles or responsibilities under this Agreement. Any subcommittees and working groups established by the JSC will dissolve when the JSC is dissolved.

(h) **Limitations on Authority of the JSC.** The JSC will have solely the roles and responsibilities assigned to it in this Article 2. For the avoidance of doubt, other than with respect to Research Program matters covered by clause (ii) of Section 2.1(c), Early Development matters covered by clause (iii) of Section 2.1(c) and CMC matters covered by clause (v) of Section 2.1(c), the JSC shall have no decision-making authority with respect to the Development or Commercialization of Therapeutics and Products. In addition, the JSC shall have no authority to amend, modify or waive compliance with this Agreement, or limit BMS' final decision-making authority with respect to the Development or Commercialization of any Therapeutic or Product as set forth in this Agreement.

**2.2 Alliance Managers.** Each of the Parties will appoint one (1) representative who possesses a general understanding of research, Development and Commercialization issues to act as its alliance manager (each, an "**Alliance Manager**") no later than thirty (30) days after the Effective Date. The Alliance Manager of a Party may only be appointed as representative of such Party in the JSC if the other Party consents in writing. The role of the Alliance Manager is to act as a primary point of contact between the Parties to assure a successful relationship between the Parties. The Alliance Managers will attend all meetings of the JSC and support the co-chairpersons of the JSC in the discharge of their responsibilities. An Alliance Manager may bring any matter to the attention of the JSC if such Alliance Manager reasonably believes that such matter warrants such attention. Each Party may change its designated Alliance Manager from time to time upon written notice to the other Party. Any Alliance Manager may designate a substitute to temporarily perform the functions of such Alliance Manager upon written notice to the other Party's Alliance Manager. Each Alliance Manager will be charged with creating and maintaining a collaborative work environment within the JSC. Each Alliance Manager also will:

- (a) be the point of first referral in all matters of conflict resolution and be involved in the resolution of disputes of the JSC as provided in Section 2.1(e);
- (b) provide a single point of communication both internally within the Parties' respective organizations and between the Parties, including during such time as the JSC is no longer constituted;
- (c) plan and coordinate any cooperative efforts under this Agreement, if any, and internal and external communications; and
- (d) take responsibility for ensuring that JSC activities, such as the conduct of required JSC meetings, occur as set forth in this Agreement and that relevant action items, if any, resulting from such meetings are appropriately carried out or otherwise addressed.

**2.3 Accounting and Financial Reporting.** Each of the Parties will appoint one (1) representative with expertise in the areas of accounting, cost allocation, budgeting and financial reporting (each, a "**Financial Representative**") no later than forty-five (45) days after the Effective Date. The Financial Representative may, at the same time, also be appointed as a representative of such Party in the JSC. The Financial Representative shall work under the direction of the JSC and directly with the Alliance Manager during the Research Term and shall provide services to and consult with the JSC thereafter, in order to address the financial, budgetary and accounting issues that arise in connection with the Research Plan or Research Program Costs. Each Financial Representative may be replaced at any time by the represented Party by providing notice thereof to the other Party. The Financial Representatives will meet as they or the JSC may agree is appropriate.

### 3. RESEARCH PROGRAM

#### 3.1 Research Program and Research Term.

- (a) **Research Program.** The Parties shall initially collaborate in carrying out a research

program to (i) advance uniQure's Lead S100A1 Therapeutic, consisting of the S100A1 Collaboration Target, in Gene Therapy, and (ii) validate three (3) additional Collaboration Targets to be designated by BMS within three (3) months of the Effective Date as Collaboration Targets and discovering and identifying Therapeutics targeting such three (3) additional Collaboration Targets, and (iii) conduct certain pre-clinical/non-clinical Development activities with respect to such Therapeutics suitable for further clinical Development for human therapeutic uses. Subject to Sections 3.3 and 3.4, BMS shall further designate [\*\*] additional Targets as Collaboration Targets pursuant to the procedure set forth in Section 3.3 and/or Section 3.4, and with respect to each such additional Target that is designated by BMS as a Collaboration Target, the Parties will collaborate on discovering and identifying Therapeutics for such Collaboration Target and conduct certain non-clinical Development activities with respect to such Therapeutics for human therapeutic uses (such research program, as amended from time to time in accordance with this Agreement, collectively being the "**Research Program**"). The Research Program will be carried out in accordance with the Research Plan.

(b) **Objective of Research Program.** The objective of the Research Program is to identify one or more Therapeutics for BMS to advance into Clinical Trials and ultimately Commercialize as Products (with an understanding for BMS to use Diligent Efforts to optimize the number of differentiated Therapeutics and Products). The Research Program will focus on activities to identify Therapeutics and will also include activities directed toward Back-up Therapeutics. The Parties will collaboratively carry out the Research Program, under the direction of the JSC and in accordance with the Research Plan and Budget.

(c) **Conduct of Research Program.** The Research Program will be conducted by each Party in good scientific manner, and in compliance with all applicable good laboratory practices and Applicable Law, to attempt to achieve efficiently and expeditiously the objectives of the Research Program. Each Party shall use reasonable efforts to ensure that its Affiliates and Third Party contractors (as applicable) likewise perform any activities under the Research Program in good scientific manner, and in compliance with all applicable good laboratory practices and Applicable Law, to attempt to achieve efficiently and expeditiously the objectives of the Research Program.

(d) **Facilities and Resources.** Each Party will maintain all laboratories, offices and other facilities at its own expense and risk necessary to carry out its responsibilities under the Research Program pursuant to the Research Plan. Each Party agrees to make its employees reasonably available at their respective places of employment to consult with the other Party on issues arising during the performance of the Research Program.

(e) **Research Term.** The Research Program will be carried out during the [\*\*] year period after the Effective Date (such initial [\*\*] year period the “**Initial Research Term**”). The Initial Research Term may be extended by the Parties pursuant to this Section 3.1(e), the “**Research Term**”). If a Party wants to extend the Research Term, it must provide the other Party a written notice of its desire to extend the Research Term at least sixty (60) days prior to the scheduled expiration of the Research Term (i.e., the applicable anniversary of the Effective Date). BMS may terminate the entire Agreement at any time (aa) for safety concerns with the uniQure Platform Technology that may adversely affect the Development of Therapeutics and/or Products as envisaged under the Research Plan in accordance with Section 13.2(b) or (bb) for scientific failure of the uniQure Platform Technology in accordance with Section 13.2(a) (e.g., failure to express the Transgene to a therapeutically meaningful level due to a feature or features of the uniQure technology platform which cannot be overcome by the use of another promoter and/or vector, or inability due to a feature or features of the uniQure technology platform (and not a mere capacity issue) to produce sufficient GMP grade virus to support the Research Plan and/or the Development Plan of a Product especially in the clinical Development phase), *provided however*, that in deviation from Section 13.2(a) the notice period for such termination shall be sixty (60) days and such notice may be provided earlier than the third anniversary of the Effective Date. In addition, BMS may terminate the Research Program for convenience at any time on or after the third anniversary of the Effective Date by

terminating the entire Agreement in accordance with Section 13.2(a). The Research Term shall automatically end upon termination of this Agreement in its entirety in accordance with Article 13 without any further notice to be provided by any of the Parties.

(f) **Consequences of Research Term Extension.** For each extension of the Research Term, subject to Section 3.5, the Joint Discovery Working Group will prepare an update to the Research Plan, which will include an updated Budget for the BMS-funded uniQure FTEs to perform the work required under such updated Research Plan and any projected Third Party Costs, for approval by the JSC.

### 3.2 Research Plan; Budget.

(a) **Research Plan.** The Research Program will be carried out in accordance with a written research plan (the “**Research Plan**”). The purpose of the Research Plan is to detail the responsibilities and activities of uniQure and BMS with respect to carrying out the Research Program. The Research Plan will include a description of the specific activities to be performed by uniQure and BMS in support of the Research Program, the number of uniQure FTEs to perform such activities in support of the Research Program, projected timelines for completion of such activities and, as applicable, provisions for the supply of Collaboration Targets and related Target Therapeutics by uniQure to BMS. The Research Plan will also include a budget for the BMS-funded uniQure FTEs (based on the number of BMS-funded uniQure FTEs and the FTE Rate) and any projected Third Party Costs (the “**Budget**”). The Budget for a Calendar Year shall be approved by the JSC prior to the commencement of such Calendar Year and the JSC shall approve any modifications to the Budget for such Calendar Year with input from the Joint Discovery Working Group and the Joint Early Development Working Group.

(b) In addition, the Budget for the first Calendar Quarter of each Calendar Year during the Research Term shall include the projected Third Party Costs for such Calendar Year. As part of this Calendar Quarter update to the Budget, the Joint Discovery Working Group and the Joint Early Development Working Group shall submit a written proposed quarterly Budget to the JSC for approval by the JSC that specifies for the coming Calendar Quarter period (i) the number of uniQure FTEs assigned to the Research Program (in accordance with Section 3.5), (ii) a summary of their activities, and (iii) a listing of the uniQure technicians and scientists comprising such FTEs, their percentage of time devoted to working on the Research Program and the allocation of their effort for activities under the Research Program. If BMS has reasonable concerns regarding any specific uniQure employee or contractor assigned to the Research Program as BMS-funded uniQure FTE, such concerns shall first be communicated to the Joint Discovery Working Group and the Joint Early Development Working Group for their consideration, and if such working groups cannot resolve BMS’ concerns, the matter will be referred to the JSC for its consideration and resolution.

(c) **Initial Research Plan.** Exhibit D contains a summary of the initial Research Plan, which covers the Lead S100A1 Therapeutic for Gene Therapy, and a projected budget for the initial Research Plan, (the “**Initial Research Plan**”). Within ninety (90) days after the Effective Date the Parties shall complete the Initial Research Plan, including the budget, for approval by the JSC. For the avoidance of doubt, the Initial Research Plan is part of the Research Plan.

(d) **Changes to the Research Plan.** The Research Plan will be reviewed and updated, as needed, with input from the Joint Discovery Working Group and the Joint Early Development Working Group on a Calendar Quarter basis, or more frequently as the JSC may decide. After each Calendar Quarter the Joint Discovery Working Group and the Joint Early Development Working Group will discuss how the actual FTE hours compared to the Budget for such Calendar Quarter and approve any deviation to the Budget for the just ended Calendar Quarter and decide on any FTE adjustment, if any, that is needed for the next Calendar Quarter. If the Joint Discovery Working Group and the Joint Early Development Working Group cannot agree on any deviation or adjustment to the Budget, the matter will be referred to the JSC for

resolution. If the JSC cannot reach consensus with respect to changes to the Research Plan or the Budget, BMS shall have final decision making authority subject to Section 2.1(d).

### 3.3 Collaboration Targets.

#### (a) Designation of Collaboration Targets.

(i) Subject to the provisions of Sections 3.3 and 3.4, in addition to the Collaboration Target identified as of the Signing Date and listed in Exhibit E, BMS shall designate nine (9) additional Targets as Collaboration Targets (each a “**New Target**”) for a total and maximum of ten (10) Collaboration Targets pursuant to the procedure set forth in this Section 3.3 and Section 3.4, with the ability to replace any of such ten (10) Collaboration Targets with a Replacement Target as set forth in Section 3.3(c).

(ii) At any time before the three (3) month anniversary of the Effective Date, BMS shall identify a first set of [\*\*] New Targets (i.e., the [\*\*] New Targets) that it wants to designate as Collaboration Targets and pay to uniQure a Target Designation Fee of [\*\*] for each such New

Target within (30) days of the date BMS designates such New Target (if such New Target is a Reserved Target), or within (30) days of the date BMS is notified by the Target Reviewer in accordance with Section 3.4 that such New Target is not an Excluded Target (if such New Target is not a Reserved Target). BMS shall designate any such New Target as a Collaboration Target promptly after becoming aware that such New Target is not an Excluded Target. If BMS is notified by the Target Reviewer in accordance with Section 3.4 that one or more of such New Targets is an Excluded Target, promptly after receipt of such notification by the Target Reviewer BMS shall designate such number of additional New Targets until the first set of [\*\*] New Targets that are not Excluded Targets has been successfully designated as Collaboration Targets, and BMS shall pay the Target Designation Fee of [\*\*] for any such additional New Target within thirty (30) days of the date BMS is notified by the Target Reviewer in accordance with Section 3.4 that such additional New Target is not an Excluded Target.

(iii) At any time before the date that is thirty (30) days after the Second Designation Date, subject to subsections (v) and (vi) below, BMS shall designate [\*\*] additional New Targets as Collaboration Targets (the “[\*\*] **New Targets**”) (i.e., [\*\*] New Targets). At least three (3) months prior to the Second Designation Date, the Joint Discovery Working Group shall begin discussing and evaluating Targets that BMS may want to consider designating as Collaboration Targets if BMS has not already designated the [\*\*] New Targets and begin working on the research plan for such Targets that BMS may want to designate as Collaboration Targets. BMS shall pay to uniQure a Target Designation Fee of [\*\*] for each such New Target within (30) days of the date BMS designates such New Target (if such New Target is a Reserved Target), or within (30) days of the date BMS is notified by the Target Reviewer in accordance with Section 3.4 that such New Target is not an Excluded Target (if such New Target is not a Reserved Target). If BMS is notified by the Target Reviewer in accordance with Section 3.4 that one or more of such New Targets is an Excluded Target, promptly after receipt of such notification by the Target Reviewer BMS shall designate such number of additional New Targets until the [\*\*] New Targets that are not Excluded Targets has been successfully designated as Collaboration Targets, and BMS shall pay the Target Designation Fee of [\*\*] for any such additional New Target within thirty (30) days of the date BMS is notified by the Target Reviewer in accordance with Section 3.4 that such additional New Target is not an Excluded Target. Notwithstanding the foregoing, if BMS fails to designate the entire [\*\*] New Targets (i.e., the [\*\*] New Targets) within thirty (30) days of the Second Designation Date, BMS shall nevertheless pay uniQure the [\*\*] Target Designation Fee for the number of remaining New Targets of the [\*\*] New Targets that have not been designated as Collaboration Targets within sixty (60) days of the Second Designation Date, and BMS may designate any of the remaining number of New Targets as Collaboration Targets up until the later of either (aa) the end of the Research Term or (bb) [\*\*] following the date on which the last Target Designation Fee for the [\*\*] New Targets was paid.

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(iv) At any time before the date that is thirty (30) days after the [\*\*], subject to subsections (v) and (vi) below BMS shall designate [\*\*] additional New Targets as Collaboration Targets (the “[\*\*] **New Targets**”) (i.e., [\*\*] New Targets). At least three (3) months prior to the Third Designation Date, the Joint Discovery Working Group shall begin discussing and evaluating Targets that BMS may want to consider designating as Collaboration Targets if BMS has not already designated the [\*\*] New Targets and begin working on the research plan for such Targets that BMS may want to designate as Collaboration Targets. BMS shall pay to uniQure a Target Designation Fee of [\*\*] for each such New Target within (30) days of the date BMS designates such New Target (if such New Target is a Reserved Target), or within (30) days of the date BMS is notified by the Target Reviewer in accordance with Section 3.4 that such New Target is not an Excluded Target (if such New Target is not a Reserved Target). If BMS is notified by the Target Reviewer in accordance with Section 3.4 that one or more of such New Targets is an Excluded Target, promptly after receipt of such notification by the Target Reviewer BMS shall designate such number of additional New Targets until the [\*\*] New Targets that are not Excluded Targets has been successfully designated as Collaboration Targets, and BMS shall pay the Target Designation Fee of [\*\*] for any such additional New Target within thirty (30) days of the date BMS is notified by the Target Reviewer in accordance with Section 3.4 that such additional New Target is not an Excluded Target. Notwithstanding the foregoing, if BMS fails to designate the entire [\*\*] New Targets (i.e., the [\*\*] New Targets) within thirty (30) days of the Third Designation Date, BMS shall nevertheless pay uniQure the \$2 million Target Designation Fee for the number of remaining New Targets of the Third Set of Three New Targets that have not been designated as Collaboration Targets within sixty (60) days of the Third Designation Date, and BMS may designate any of the remaining number of New Targets as Collaboration Targets up until the later of either (aa) the end of the Research Term or (bb) [\*\*] following the date on which the last Target Designation Fee for the [\*\*] New Targets was paid. If BMS designates any of the remaining number of New Targets during the [\*\*] year of the Research Term, the Parties will mutually agree to extend the Research Term for at least [\*\*] year.

(v) Notwithstanding the foregoing, by mutual written agreement, the Parties may postpone the Second Designation Date and/or the Third Designation Date, whereupon the date by which the [\*\*] New Targets and the [\*\*] New Targets, respectively, should be designated shall be thirty (30) days after the postponed date, and the Target Designation Fee for any undesignated New Target shall be due within sixty (60) days of such postponed date.

(vi) For the avoidance of doubt and notwithstanding anything to the contrary in this Agreement, (a) BMS does not have to designate [\*\*] as a New Target at any one time, (b) BMS is obligated to designate [\*\*] New Targets pursuant to subsection (ii) above, and (c) with respect to the [\*\*] New Targets and the [\*\*] New Targets, if BMS fails to designate [\*\*] of New Targets in the [\*\*] New Targets and/or the [\*\*] New Targets prior to the respective due dates set forth in subsections (iii) and (iv) above (or the due dates set forth in subsection (v) above if the Second Designation Date or the Third Designation Date is postponed pursuant to subsection (v) above), BMS shall be obligated to [\*\*] for each such New Target as if BMS had timely designated such New Target prior to the respective due dates set forth in subsections (iii) and (iv) above (or the due dates set forth in subsection (v) above), BMS shall [\*\*] for its failure to designate the entire number of New Targets in the [\*\*] New Targets and/or the [\*\*] New Targets prior to the respective due dates set forth in subsections (iii) and (iv) above (or the due dates set forth in subsection (v) above), and BMS shall [\*\*] if it does not thereafter designate the entire number of New Targets in the [\*\*] New Targets and/or the [\*\*] New Targets. [\*\*] of the corresponding [\*\*] shall be made within the respective due dates set forth in subsections (ii) to (v) above.

(vii) Once a Target becomes a New Target, the Research Plan shall be amended to include such New Target, a Budget for such New Target shall be approved by the JSC, and work on such New Target under the Research Plan shall commence no later than the date mutually agreed by the Parties

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for work to commence, unless such New Target is replaced by a Replacement Target prior to the commencement of such work, in which case work on such Replacement Target shall commence no later than the date mutually agreed by the Parties for work to commence on such Replacement Target. Until a Target becomes a New Target and work on such New Target commences under the Research Plan, BMS shall have no obligation with respect to the research,



(b) **Target Reservation.**

(i) During the Research Term, BMS shall be entitled to designate a number of Targets as reserved Targets, with each such Target so designated being a “**Reserved Target**”. Each such Target to be designated as Reserved Target shall be designated in accordance with and subject to the Excluded Target process as set forth in Section 3.4. The maximum number of Reserved Targets shall be [\*\*] at the Effective Date and shall decrease following the designation of New Targets by BMS pursuant to Section 3.3(a) as follows: After the designation of one or more New Targets of the first set of New Targets pursuant to Section 3.3(a)(i), the maximum number of Reserved Targets shall be [\*\*]. After the designation of or, if no designation is undertaken in due time, upon expiration of BMS’ right to designate one or more New Targets of the [\*\*] New Targets pursuant to Section 3.3(a)(iii), the maximum number of Reserved Targets shall be [\*\*]. After the designation of one or more New Targets of the [\*\*] New Targets pursuant to Section 3.3(a)(iv), the maximum number of Reserved Targets shall be [\*\*]. Upon expiration of BMS’ right to designate one or more New Targets of the [\*\*] New Targets pursuant to Section 3.3(a)(iv), any remaining Reserved Targets [\*\*], [\*\*]. Together with its designation of the relevant New Target that will result in a reduction of the maximum number of Reserved Targets, BMS shall specify which of the Reserved Targets shall cease to be designated as Reserved Targets. For the avoidance of doubt, a Target shall not become an Excluded Target so long as such Target is a Reserved Target or a Collaboration Target and any Reserved Target ceasing to be designated as a Reserved Target due to a reduction of the maximum number of Reserved Targets shall be a Target and shall be subject to the exclusivity undertakings pursuant to Article 11.

(ii) BMS may replace designated Reserved Targets up to the respective maximum number of Reserved Targets at any time during the Research Term in accordance with and subject to the Excluded Target process as set forth in Section 3.4. Any Reserved Target ceasing to be designated as a Reserved Target due to a replacement of such Reserved Target by another Target shall be a Target and shall be subject to the exclusivity undertakings pursuant to Article 11.

(iii) For so long as a Target is a Reserved Target, uniQure agrees that it shall not [\*\*]:

Subject to the terms and conditions of this Agreement, and without limiting the rights granted to BMS under this Agreement, if [\*\*] with uniQure regarding a possible [\*\*] with respect to any [\*\*], uniQure shall notify BMS no less than [\*\*] days prior to [\*\*]. After uniQure provides such a notice to BMS, BMS shall have a period of [\*\*] to designate such [\*\*]. If BMS does not so designate [\*\*], uniQure shall be permitted, for a period of [\*\*] following the end of the BMS designation period, to [\*\*]. uniQure will provide written notice to BMS notifying BMS [\*\*]. If uniQure fails to [\*\*].

If uniQure wants to initiate an internal program for a Reserved Target or any Variant thereof on its own, uniQure shall [\*\*] of its intention to initiate such internal program. If uniQure then initiates such internal program for such Reserved Target or any Variant thereof, uniQure shall [\*\*], except as described below. Where uniQure initiates an internal program for a Reserved Target or any Variant thereof, as the case may be, [\*\*], then once uniQure has [\*\*], as the case may be, of interest in [\*\*], uniQure shall [\*\*] that it has [\*\*] and [\*\*] and shall provide [\*\*] for such internal program. BMS shall have [\*\*] days after

receiving all such [\*\*] to notify uniQure [\*\*], as the case may be, of such internal program as a [\*\*]. If BMS notifies uniQure that it [\*\*], as the case may be, as a [\*\*], (A) BMS shall [\*\*] in conducting the internal program for such Reserved Target or such Variant, as the case may be, up until [\*\*] in accordance with the foregoing sentence that such internal activities have been completed, (B) BMS shall [\*\*] that have been achieved by or on behalf of [\*\*] in conducting the internal program for such Reserved Target or such Variant, as the case may be, up until the [\*\*] in accordance with the foregoing sentence that such internal activities have been completed ([\*\*]), (C) the [\*\*] shall be amended to include [\*\*], as the case may be, and (D) uniQure shall only [\*\*], as the case may be, as part of the [\*\*]. If, however, BMS notifies uniQure that [\*\*], as the case may be, as a [\*\*] or fails to provide any written notice to uniQure during such thirty [\*\*] period, such [\*\*], as the case may be, shall become an [\*\*], and uniQure shall have the right to [\*\*] regarding a [\*\*], and to [\*\*].

Notwithstanding anything to the contrary in this Section 3.3(b), in no event may uniQure [\*\*] as a [\*\*] for more than a total of [\*\*] Reserved Targets and/or Variants thereof in any [\*\*] period (i.e., the limit of [\*\*] Reserved Targets and/or Variants thereof can be achieved by (a) [\*\*] Reserved Targets and/or Variants thereof of [\*\*], (b) [\*\*] Reserved Targets and/or Variants thereof for which [\*\*], or (c) [\*\*]).

(c) **Adding or Replacing a Collaboration Target.**

(i) **Adding a Collaboration Target.** BMS shall have the right to designate any Reserved Target as a New Target. In the case where BMS desires to designate a Target as a New Target where such Target has not previously been designated as a Reserved Target, BMS shall follow the Excluded Target process set forth in Section 3.4, where the process is applied to such new proposed Target. For clarity, BMS may designate any Reserved Target as a Collaboration Target by providing written notice to the JSC without following the Excluded Target process set forth in Section 3.4 (since such process has already been conducted for such Reserved Target as set forth in subsection (b) above). For the avoidance of doubt, the maximum number of Collaboration Targets designated under this Agreement shall be ten (10) and BMS shall not have the right to designate any additional Targets as Collaboration Targets where such designation would lead to the maximum number of ten (10) Collaboration Targets being exceeded; *provided, however*, no [\*\*] shall count towards the total and maximum of [\*\*] Collaboration Targets, but shall be counted instead of the [\*\*] only. By way of illustration and not limitation, if [\*\*] New Targets have been designated by BMS as Collaboration Targets so there are a total of [\*\*] Collaboration Targets, and the [\*\*] New Target designated by BMS as a Collaboration Target (i.e., the [\*\*] Collaboration Target) is replaced by a Replacement Target, such Replacement Target shall not count as the [\*\*] Collaboration Target but will instead be considered the [\*\*] New Target designated by BMS (i.e., the [\*\*] Collaboration Target) for purposes of determining the total and maximum of [\*\*] Collaboration Targets.

(ii) **Replacing a Collaboration Target.** During the Research Term, BMS shall be able to replace any of the then-current Collaboration Targets with a new Target (a “**Replacement Target**”) that may be a Reserved Target or another new Target (in accordance with and subject to the Excluded Target process as described in Section 3.4); *provided however*, that with respect to all but the [\*\*], BMS may replace a Collaboration Target for [\*\*], and with respect to [\*\*], BMS may replace a Collaboration Target [\*\*] for legal, Third Party intellectual property or scientific reasons as well as for Safety Reasons. Subject to the foregoing limitations, BMS shall have the right to replace an existing Collaboration Target with a Replacement Target as set forth above at any time during the Research Term where such Replacement Target is a Reserved Target, by providing written notice to the JSC without following the Excluded Target process set forth in Section 3.4 (since such process has already been conducted for such Reserved Target as set forth in

subsection (b) above). In the case where BMS desires to replace an existing Collaboration Target with a Replacement Target that has not previously been designated as a Reserved Target, BMS shall follow the Excluded Target process set forth in Section 3.4, where the process is applied

to such new proposed Replacement Target. For the avoidance of doubt, no Target Designation Fee is due or payable for a Replacement Target.

(d) **Disposition of a Replaced Target.**

(i) Upon replacement of a previously designated Collaboration Target with a Replacement Target, such [\*\*] shall no longer be [\*\*].

(ii) If the Replaced Target is a BMS Proprietary Target, [\*\*] to such Replaced Target or any [\*\*]. If uniQure wants to [\*\*] on such Replaced Target or any [\*\*] and such Replaced Target was not replaced by a Replacement Target for any Safety Reason, [\*\*].

(iii) If the Replaced Target does not fall within the scope of Section 3.3(d)(ii) and was not replaced by a Replacement Target for any Safety Reason, it shall [\*\*], and the provisions of [\*\*] shall apply to [\*\*], and in applying [\*\*] to any such Replaced Target, each reference to “Reserved Target” shall be replaced by “Replaced Target”, *provided however*, [\*\*], such Replaced Target shall [\*\*] but BMS shall be [\*\*] on a [\*\*] with respect to such Replaced Target, and the provisions of [\*\*] shall apply to such Replaced Target and the Therapeutics and Products for such Replaced Target.

(iv) Notwithstanding Section 3.3(d)(iii), if the Replaced Target does not fall within the scope of Section 3.3(d)(ii) and was replaced by a Replacement Target for any Safety Reason, then [\*\*] on a [\*\*] with respect to such Replaced Target, and the provisions of [\*\*] shall apply to such Replaced Target and the Therapeutics and Products for such Replaced Target.

(e) **Declined Targets.**

(i) Where any Target that is proposed for the Joint Discovery Working Group to discuss as a potential Collaboration Target becomes a Declined Target, and if BMS notifies uniQure that [\*\*], it shall be [\*\*] (in particular, it shall [\*\*] Reserved Targets pursuant to [\*\*] and it shall only be [\*\*] as long as [\*\*]), and the provisions of [\*\*] shall apply to each such Declined Target, and in applying [\*\*] to any such Declined Target, each reference to “Reserved Target” shall be replaced by “Declined Target”, *provided however*, that notwithstanding [\*\*], if a particular Target that became a Declined Target eventually becomes a Replaced Target for the first time, such [\*\*] but BMS shall [\*\*] on a [\*\*] with respect to such Declined Target / Replaced Target, and the provisions of [\*\*] shall apply to such Declined Target / Replaced Target and the Therapeutics and Products for such Replaced Target.

(ii) Notwithstanding the foregoing, if such Declined Target / Replaced Target was replaced by a Replacement Target for any Safety Reason, then BMS shall be [\*\*] on a [\*\*] with respect to such Declined Target / Replaced Target, and the provisions of [\*\*] shall apply to such Declined Target / Replaced Target and the Therapeutics and Products for such Declined Target / Replaced Target.

### 3.4 **Excluded Target Process; Designation of Reserved Targets and Collaboration Targets.**

(a) **Target Reviewer.**

(i) In the case where BMS desires to add a new Reserved Target (up to the maximum number of Reserved Targets pursuant to Section 3.3(b)), replace an existing Reserved Target, or propose a new Target as Collaboration Target (including as Replacement Target), BMS shall notify uniQure through the Joint Discovery Working Group. Within fifteen (15) Business Days after such notification, uniQure shall [\*\*](the “**Target Reviewer**”) with an updated [\*\*] (which shall include [\*\*] information for each [\*\*]). Within [\*\*] Business Days after uniQure has provided the Target Reviewer with such updated [\*\*], BMS shall provide to the Target Reviewer the [\*\*] to become a Reserved Target or Collaboration Target. Within [\*\*] Business Days thereafter, the Target Reviewer shall [\*\*]. If the proposed Target is not

an Excluded Target, BMS shall [\*\*], as the case may be, in accordance with Section [\*\*]. Accordingly, any Target that is not an Excluded Target shall be [\*\*]. BMS shall [\*\*].

(ii) In the case where BMS is considering a Target as a potential Collaboration Target or potential Reserved Target and does [\*\*] before determining whether such Target is an Excluded Target, then BMS shall [\*\*] within [\*\*] Business Days after such request [\*\*]. Within [\*\*] Business Days after uniQure has provided the Target Reviewer with such updated Excluded Target List, BMS shall [\*\*]. Within [\*\*] Business Days thereafter, the Target Reviewer shall [\*\*]. If the proposed Target is not an Excluded Target, BMS shall [\*\*], as the case may be, in accordance with [\*\*]. Accordingly, any Target that is not an Excluded Target shall [\*\*]. BMS shall [\*\*] and shall not be obliged to [\*\*].

(b) **Consultation with uniQure; Designation of New Reserved Target or Collaboration Target.** Promptly after the Target Reviewer has [\*\*], BMS shall [\*\*], including [\*\*]. The [\*\*] shall [\*\*], as the case may be; *provided however*, that BMS shall [\*\*] with respect to the [\*\*], as the case may be. BMS may [\*\*], as the case may be, by [\*\*], and in conjunction with the [\*\*], uniQure shall [\*\*].

(c) **Alternative Procedure.** For purposes of the designation of any particular Target as a Collaboration Target, the Parties may [\*\*] and instead allow [\*\*] and BMS to [\*\*].

(d) **Interest in Targets.** The Parties agree and acknowledge that BMS, without any prejudice to its rights and obligations related to the designation of Collaboration Targets or Reserved Targets pursuant to Section 3.3, is entitled to provide [\*\*]. Upon receipt [\*\*]uniQure, before either (i) [\*\*] or (ii) [\*\*], shall first [\*\*]. If [\*\*] within [\*\*] months after [\*\*], [\*\*].

### 3.5 **Research Staffing and Funding During the Research Term.**

(a) **Funded uniQure FTEs; FTE Rate.** Subject to Section 3.5(b), BMS will fund at the FTE Rate, and uniQure will provide the number of uniQure FTEs to perform activities in support of the Research Program, in accordance with the then-current Research Plan, and in accordance with this Section 3.5. Throughout the Research Term and subject to the limitations set forth in Section 3.5(b), uniQure shall assign no less than the number of scientist FTEs in accordance with this Section 3.5 to perform the work set forth in the then-current Research Plan. The qualifications, professional skills and expertise levels of such FTEs shall be appropriate to the scientific objectives of the Research Program. The FTE Rate for the initial Research Year shall be [\*\*] per FTE per year, and such rate shall increase by [\*\*] for each subsequent Research Year during the Research Term. For the avoidance of doubt, nothing in this Agreement herein shall be considered to establish an employment relationship between BMS and the uniQure FTEs funded by BMS pursuant to this Agreement.

(b) **Changes to the Number of Funded FTEs.** If the activities contemplated by the Research Plan at any time do not reasonably justify the number of uniQure FTEs allocated to the Research Program, the Parties will work in good faith to mutually agree to modify the scope of the Research Plan or adjust the number of BMS-funded uniQure FTEs. The number of uniQure FTEs to be funded by BMS and provided by uniQure in support of the conduct of the Research Program may be increased or decreased by the JSC in accordance with changes in the Research Program and Research Plan and shall be specified for each Calendar Quarter in the Budget as set forth in Section 3.2(a). Any changes to the Research Plan and assignment and allocation of work to be performed by the BMS-funded uniQure FTEs shall require the approval of the JSC taking into consideration any recommendations of the Joint Discovery Working Group; *provided however*, that if the JSC is unable to reach consensus, BMS shall have final decision making authority, subject to Section 2.1(d). For clarity, the number of BMS-funded uniQure FTEs will be consistent with the amount of work required under the Research Plan. At least sixty (60) days prior to the beginning of any extension of the Research Term, the JSC shall determine the number of uniQure FTEs to be provided and funded by BMS to perform the Research Program during such extended period of the Research Term;

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*provided however*, that if the JSC is unable to reach consensus, BMS shall have final decision making authority with respect to the number of BMS funded uniQure FTEs, subject to Sections 2.1(d) and this Section 3.5.

(c) **FTE Funding; Research Program Costs.** BMS will be responsible for the payment to uniQure for the BMS-funded uniQure FTEs specified in the Budget (to the extent that such FTEs are actually provided by uniQure) and for the Third Party Costs specified in the Budget (to the extent incurred by uniQure), as may be amended in accordance with Section 3.2 and this Section 3.5 (such FTE payment and Third Party Costs being the “Research Program Costs”).

(d) **Payments of FTE Funding.** The number of BMS-funded uniQure FTEs shall be established in accordance with Section 3.5(a) and (b), and BMS shall fund such uniQure FTEs at the FTE Rate in accordance with the Budget. Such FTE payment obligation of BMS will be subject to uniQure providing such qualified FTE scientists. Such FTE payment obligation shall be payable in arrears on a Calendar Quarter-to-Calendar Quarter basis with such invoice being due within [\*\*] days following the end of such Calendar Quarter. For clarity, such payments for the first Calendar Quarter and the last Calendar Quarter of the Research Term shall be based on the portion of the Calendar Quarter within the Research Term. Such invoice for such FTE payment obligation shall be payable within [\*\*] days after BMS receives such invoice. Accompanying the invoice, uniQure will provide BMS with a report of the number of FTEs assigned to the Research Program during the invoiced period with a summary of their activities. uniQure shall report to BMS a listing of the uniQure scientists comprising such FTEs and their percentage of time devoted to working on the Research Program.

(e) **Invoices for Third Party Costs.** uniQure shall invoice BMS for the Third Party Costs approved within the Budget and incurred by uniQure for a given Calendar Quarter within [\*\*] days following the end of such Calendar Quarter (such invoice shall include supporting documentation for such Third Party Costs and be sent to BMS’ Financial Representative or otherwise as specified in writing by BMS). Such invoice for such Third Party Costs reimbursable by BMS shall be payable within [\*\*] days after BMS receives such invoice.

**3.6 Responsibility for Expenses for Conduct of Research Program.** Except as otherwise set forth in this Agreement or as may be otherwise specifically agreed to in writing by uniQure and BMS, each Party shall be responsible for its own costs and expenses that it incurs in connection with the conduct of the Research Program. For clarity, subject to the funding by BMS of the Research Program Costs in accordance with the Budget, uniQure shall be responsible for all costs that it incurs in the conduct of the Research Program.

**3.7 Research Program Records.** Each Party will maintain complete and accurate records of all work conducted in the performance of the Research Program and all results, data, inventions and developments made in the performance of the Research Program. Such records will be in sufficient detail and in good scientific manner appropriate for patent and regulatory purposes. uniQure shall maintain appropriate records sufficient to document the work performed by each of the individuals comprising the FTEs working in support of the Research Program and the time such individuals spent working in support of the Research Program. uniQure shall provide copies of all requested records (within sixty (60) days of such request), to the extent reasonably required for the performance of BMS’ rights and obligations under this Agreement; *provided however*, that BMS shall maintain such records and the information of uniQure in confidence in accordance with Article 12 and shall not use such records or information except to the extent otherwise permitted by this Agreement. In order to protect the Parties’ Patent rights under U.S. law and any other Applicable Law in any inventions conceived or reduced to practice during or as a result of the Research Program, each Party shall require such individuals to record all inventions generated by them in standard laboratory notebooks (paper or electronic) or other suitable means that are dated and corroborated by non-inventors on a regular, contemporaneous basis.

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**3.8 Disclosure of Results of Research Program.** The results of all work performed by a Party as part of the Research Program shall be promptly disclosed to the other Party in a reasonable manner as such results are obtained. uniQure and BMS will provide reports and analyses at each JSC meeting, and more frequently upon reasonable request by the JSC, detailing the current status of the Research Program, including the utilization of the uniQure FTE resources. Within sixty (60) days following the end of each Calendar Quarter, uniQure and BMS shall each exchange and provide to the JSC a written report summarizing in reasonable detail the work performed by it under the Research Program and results achieved during the preceding Calendar Quarter. In addition, upon reasonable request by a Party, the other Party will make presentations to the JSC of its activities related to the Therapeutics and Products to inform such Party of the details of the work done in the performance of the Research Program. The results, reports, analyses and other information regarding the Research Program disclosed by one Party to the other Party pursuant hereto may be used only in accordance with the rights granted and other terms and conditions under this Agreement. Upon reasonable request by BMS, for purposes of supporting the Development of Products (including supporting or maintaining the Regulatory Approval for Products), subject to and without limiting the other terms and conditions of the Agreement, uniQure

shall provide BMS with additional data, results and other Information with respect to the work performed by uniQure in the performance of the Research Program. Any reports required under this Section 3.8 may take the form of and be recorded in minutes of the JSC that will contain copies of any slides relating to the results and presented to the JSC. In addition, at BMS' request uniQure will transfer (within sixty (60) days of such request) to BMS all data, results, and information related to testing and studies undertaken as part of the Research Program, or prior to the Effective Date with respect to any Collaboration Target or Therapeutic (including analytical test results and non-clinical pharmacology and safety data) in the possession and Control of uniQure to the extent such data, results or information are necessary or reasonably useful for the conduct of the Research Program or for the continued Development and Commercialization of Therapeutics and Products.

**3.9 Research Efforts.** Each Party shall use Diligent Efforts to perform the Research Program, including its responsibilities under the Research Plan. For clarity, it is understood and acknowledged that Diligent Efforts to perform the Research Program may include staging the work on different Collaboration Targets as specified in and in accordance with the Research Plan.

**3.10 Materials Transfer.** This Section 3.10 shall not apply to the supply of Therapeutics and Raw Materials pursuant to Article 6, which shall be governed by Article 6.

(a) In order to facilitate the Research Program, either Party may provide to the other Party certain materials (other than samples of Target Therapeutics or Raw Materials as provided under Sections 6.2 and 6.4) for use by the other Party in furtherance of the Research Program and the Development and Commercialization of Therapeutics and Products. All such materials (including, as applicable, any progeny, expression products, mutants, replicates, derivatives and modifications thereof) shall be used by the receiving Party in accordance with the terms and conditions of this Agreement solely for purposes of performing its rights and obligations under this Agreement, and the receiving Party shall not transfer such materials (including, as applicable, any progeny, expression products, mutants, replicates, derivatives and modifications thereof) to any Third Party unless expressly contemplated by this Agreement (including the Research Plan) or upon the written consent of the supplying Party. As set forth in the Research Plan, each Party shall provide the other Party with samples of such materials for use by the other Party in accordance with the terms and conditions of this Agreement (including the Research Plan). All Information related to such materials shall be Confidential Information of the providing Party. All such materials must be used with prudence and appropriate caution in any experimental work, since all of their characteristics may not be known.

(b) Any materials provided by BMS to uniQure (including, as applicable, any progeny, expression products, mutants, replicates, derivatives and modifications thereof) shall be returned to BMS (or destroyed as may be requested by BMS in writing) promptly following the end of the Research Term or

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earlier upon request by BMS. If uniQure develops any assays used in the Research Program, upon request by BMS, uniQure shall transfer to BMS the materials and Information to enable BMS to use such assays in support of BMS' research and Development activities with respect to the Collaboration Targets.

(c) Any materials provided by uniQure to BMS (including, as applicable, any progeny, expression products, mutants, replicates, derivatives and modifications thereof) shall be returned to uniQure (or destroyed as may be requested by uniQure in writing) promptly following termination of such Collaboration Target, if such materials were specifically provided for a particular Collaboration Target, or, otherwise, upon request by uniQure or termination of this Agreement by BMS pursuant to Section 13.7(f).

**3.11 Subcontracting.** Except as required to have manufactured any Therapeutics or Raw Materials required to be provided by uniQure under this Agreement or as provided in the Research Plan or as may be specifically permitted in writing by the JSC, or otherwise agreed to in advance in writing by BMS, uniQure shall not (sub)contract any of the work for which it is responsible in the performance of the Research Program except to any of its Affiliates, unless uniQure has obtained the prior written consent of BMS (which may occur through the JSC), which consent shall not be unreasonably withheld, conditioned or delayed. In the case of any permitted (sub)contracting of Research Program activities by a Party to a Third Party, such Third Party must have entered into a written agreement with such Party that includes terms and conditions protecting and limiting use and disclosure of Confidential Information and Know-How at least to the same extent as under this Agreement; *provided however*, that the term of such Third Party's obligations regarding the use and disclosure of Confidential Information and Know-How shall be as long as can be reasonably negotiated with such Third Party, but in any event no less than [\*\*] years after the date of disclosure to the Third Party. Each Party is responsible for compliance by such Third Party with the applicable terms and conditions of this Agreement in the same way and to the same extent as such Party. Any agreement between uniQure and a Third Party for the Third Party to conduct Research Program activities for uniQure as a subcontractor shall be subject to the prior review and approval by BMS.

**3.12 Animal Testing.** In order to assure the appropriate care and use of animals used in the performance of the Research Program by uniQure, uniQure agrees to the following:

(a) If uniQure or its relevant Affiliate is AAALAC accredited, it will follow procedures established as the basis of that accreditation. uniQure represents and covenants that it will, and will procure that its relevant Affiliate will, use all reasonable efforts to maintain such AAALAC accreditation during the Research Term. Further, upon request by BMS, uniQure will provide BMS with a copy of the most recent accreditation letter and annual report. If during the course of the Research Program uniQure or its relevant Affiliate loses its accreditation or receives any notice, warning or reprimand from AAALAC or any Governmental Authority related to animal care and use, uniQure will promptly notify BMS in writing.

(b) If uniQure or its relevant Affiliate is not AAALAC accredited or loses its AAALAC accreditation at any time during the Research Term, it will comply with **Exhibit K**.

(c) Whenever possible, live animals used as part of the Research Program should remain the property of the applicable contract facility. Upon reasonable advance notice during the Research Term, representatives of BMS shall have the right to inspect the research facilities and to audit the care, treatment and use of the animals used in the Research Program. This includes the right to review any correspondence with or reports from governmental agencies or accrediting organizations responsible for animal welfare or quality assurance

(d) At least two (2) months after the Signing Date or if later, by the date that is two (2) months before uniQure will use HDU and/or any other Third Party for any animal testing under the Research Plan, uniQure shall notify BMS in writing that it wants to utilize HDU and/or such other Third Party for such animal testing and whether or not HDU and/or such other Third Party is AAALAC accredited, or the equivalent.

**3.13 Information Transfer to BMS.** Without limiting the licenses and other rights and obligations under this Agreement (including the rights granted to BMS under Article 7 and uniQure's obligations under Section 3.7), upon request by BMS, uniQure shall deliver, and cause its Affiliates to deliver, to BMS uniQure Know-How in its possession and Control for the sole purposes of (i) work by BMS in support of the Research Program, (ii) supporting the conduct of the Research Program, and (iii) the Development and Commercialization of Therapeutics and Products by BMS (collectively, the "**Know-How Transfer Purposes**"). In addition, uniQure shall promptly disclose to BMS' Patent Contact any new uniQure inventions for which uniQure has determined it desires to seek a Product Specific Patent. uniQure shall provide reasonable consultation and assistance for the purpose of transferring to BMS uniQure Know-How to the extent necessary or reasonably useful for the Know-How Transfer Purposes. With respect to the transfer to BMS of such uniQure Know-How (and any consultation and assistance to be provided by uniQure with respect thereto), (x) the Parties shall agree in advance in writing as to the number of uniQure FTE-hours to be utilized by uniQure for such purpose and any out-of-pocket expenses (including travel expenses) that are agreed to be incurred by uniQure for such purpose and (y) periodically following the transfer of any such uniQure Know-How and providing such requested consultation and assistance, BMS shall reimburse uniQure for the agreed upon out-of-pocket expenses incurred by uniQure and for such agreed upon FTE-hours (as incurred by uniQure) to the extent in excess of the FTE Threshold, applying the then-applicable FTE Rate. For the avoidance of doubt, this Section 3.13 does not obligate uniQure to transfer any uniQure Manufacturing Know-How to BMS other than such uniQure Manufacturing Know-How that is necessary for BMS to carry out its obligations under this Agreement.

**3.14 Use of Third Parties.** BMS may retain Third Parties to conduct research consistent with BMS' license under Section 7.1(c) or to perform Development activities, subject to the terms of this Agreement. Any such Third Parties shall be subject to written agreements containing confidentiality and non-use obligations consistent with those set forth in this Agreement; *provided however*, that the term of such Third Party's obligations regarding the use and disclosure of Confidential Information and Know-How shall be as long as can be reasonably negotiated with such Third Party, but in any event no less than **[\*\*]** years after the date of disclosure to the Third Party. BMS shall remain responsible and liable for the performance by its Affiliates or permitted Third Party contractors of those of its obligations under this Agreement that it (sub)licenses or delegates to an Affiliate or Third Party contractor. Notwithstanding the foregoing, in the event BMS wants to retain a Third Party to conduct CMC activities, the prior written consent of uniQure will be needed, such consent not to be unreasonably withheld, conditioned or delayed.

**3.15 Inspection of Records.** Upon reasonable prior notice of not less than ninety (90) days, uniQure shall permit an independent nationally recognized certified public accounting firm (subject to obligations of confidentiality), appointed by BMS and reasonably acceptable to uniQure, to inspect the applicable records of uniQure to verify the Research Program Costs (including the level of FTE effort); *provided however*, that such inspection shall not occur more often than once per Calendar Year (for the current and preceding Calendar Year), unless a material error is discovered as part of such inspection in which case BMS shall have the right to conduct a more thorough inspection for such period. Any inspection conducted under this Section 3.15 shall be at the expense of BMS. Any overpayment by BMS to uniQure shall be credited against future amounts due by BMS to uniQure. Any underpayment by BMS shall be paid in the next quarterly payment to uniQure or within forty-five (45) days, whichever is later.

## 4. DEVELOPMENT AND REGULATORY MATTERS

### 4.1 Development.

(a) **Development Responsibilities.** Except for uniQure's rights and responsibilities in the conduct of the Research Program and in connection with the JSC and the subcommittees and working groups established by the JSC, and without prejudice to the diligence obligations of BMS provided herein, as between BMS and uniQure, BMS shall have the sole right and responsibility for the Development of

Therapeutics or Products in the Field in the Territory during the Term at its own cost and expense (including responsibility for all funding, resourcing and decision-making). In addition, for clarity, and except for any activities expressly assigned to uniQure hereunder, following a Therapeutic being designated as an ECN by BMS, BMS will assume control of all further Development of such ECN and will be solely responsible, including responsibility for all funding, resourcing and decision-making, for all further pre-clinical and clinical development and manufacturing (except as otherwise provided in Article 6, the Supply Agreement and the statements of work under the Supply Agreement) activities with respect to such ECN, consistent with its diligence obligations.

(b) The Development of Therapeutics and Products in the Field by BMS, including its Affiliates and Sublicensees, shall be conducted pursuant to a development plan that shall include both (i) **[\*\*]** and (ii) **[\*\*]** (the "**Development Plan**"). The Development Plan shall provide an overview of **[\*\*]** for the Development of the applicable **[\*\*]** and does not have to be **[\*\*]**. The initial Development Plan (the "**Initial Development Plan**") shall be provided by BMS to uniQure through the JSC within **[\*\*]** months before the first expected filing of an IND for a Product. For the avoidance of doubt, the Initial Development Plan is part of the Development Plan.

(c) **Updates to the Development Plan.** Following BMS' submission of the Initial Development Plan, **[\*\*]**, BMS shall provide uniQure through the JSC with annual updates to the Development Plan. Such annual updated Development Plan shall take into account completion or cessation of Development activities or commencement of new Development activities. With regard to **[\*\*]**, in the event BMS makes a **[\*\*]**, BMS, through its Alliance Manager, will **[\*\*]**. In the event **[\*\*]**, the Alliance Managers will **[\*\*]**. For the avoidance of doubt, any such **[\*\*]**. For further avoidance of doubt, the fact that **[\*\*]**, shall be without any prejudice to **[\*\*]** pursuant to this Agreement, **[\*\*]**.

(d) **Development Records.** BMS shall prepare and maintain and shall cause its Affiliates and Sublicensees to prepare and maintain reasonably complete and accurate records regarding the Development of Therapeutics and Products in the Field in the Territory.

(e) **Clinical Development Forum.** BMS shall keep uniQure informed of BMS' clinical Development activities through meetings of the Clinical Development Forum. After **[\*\*]**, the Clinical Development Forum will meet **[\*\*]**.

**4.2 Regulatory Matters for Product.** Except for uniQure's rights and responsibilities in the conduct of the Research Program and in connection with the JSC and the subcommittees and working groups established by the JSC, as between BMS and uniQure, BMS shall have sole responsibility and decision-making authority with respect to regulatory matters for Therapeutics and/or Products (including the content of any regulatory filing or dossier, pharmacovigilance reporting, labeling, safety, and the decision to file or withdraw any BLA, MAA or JNDA or to cease or suspend any

Clinical Trial). BMS shall have sole responsibility for preparing and submitting all Regulatory Materials for Products in the Field in the Territory, including preparing, submitting and holding all INDs, BLAs, MAAs and JNDAs for Products. Notwithstanding the foregoing, uniQure shall be responsible for preparing the CMC portion of any regulatory filing or dossier for Products. uniQure shall cooperate fully with BMS and provide to BMS all Information Controlled by uniQure, in each case as may be reasonably requested by BMS, in order to prepare or support any Regulatory Materials for Products in the Field in the Territory and interactions with any Regulatory Authority in connection with Development or Regulatory Approval of Products. BMS will own all Regulatory Materials for Products and all such Regulatory Materials shall be submitted in the name of BMS (or its Affiliate or Sublicensee, as applicable).

**4.3 Notice of Regulatory Action.** If any Regulatory Authority takes or gives notice of its intent to take any regulatory action with respect to any activity of a Party related to the Research Program or otherwise relating to Therapeutics or Products, then such Party shall promptly notify the other Party of such

contact, inspection or notice or action. The Joint Regulatory Working Group shall review and comment on any such responses to Regulatory Authorities that pertain to the Therapeutics or Products; *provided however*, that BMS shall have the final decision making authority with respect to such responses to the extent relating to the Therapeutics and/or Products.

**4.4 No Use of Debarred Person.** During the Term, each Party agrees that it will not use any employee or consultant that is debarred by any Regulatory Authority or, to the best of such Party's knowledge, is the subject of debarment proceedings by any Regulatory Authority. If a Party learns that any employee or consultant performing on its behalf under this Agreement has been debarred by any Regulatory Authority, or has become the subject of debarment proceedings by any Regulatory Authority, such Party will promptly notify the other Party and will prohibit such employee or consultant from performing on its behalf under this Agreement.

**4.5 BMS Diligence; Standards of Conduct.** With respect to each Collaboration Target, BMS, by itself or through its Affiliates and Sublicensees, shall use Diligent Efforts to (a) [\*\*]; and (b) [\*\*]. BMS shall perform, and shall use reasonable efforts to ensure that its Affiliates, Sublicensees and Third Party contractors perform, its Development activities with respect to the Product in good scientific manner, and in compliance in all material respects with the requirements of Applicable Law. A breach of BMS' obligation to use Diligent Efforts with respect to a Collaboration Target under this Section 4.5 shall have occurred if no Development activity pursuant to the Research Plan or the Development Plan has been undertaken with respect to a Therapeutic for such Collaboration Target by BMS, its Affiliates or Sublicensees for a period of at least [\*\*] months, unless the lack of such Development activity is due to a Safety Reason or to any reason beyond BMS' reasonable control, including a regulatory hold, the inability of uniQure to deploy an adequate number of FTEs to support the Research Program or the Development activities for which uniQure is responsible for, or the inability of uniQure to be able to supply the Therapeutic for such Collaboration Target, with any such period not to be counted towards such [\*\*] consecutive period.

**4.6 Companion Diagnostics.** BMS and its Affiliates may elect to Develop and Commercialize, either alone or in conjunction with any Third Party, one or more Companion Diagnostics in relation to the Products. The Parties will discuss in good faith the role and responsibility that uniQure may have in connection with the Development of such Companion Diagnostics. BMS shall [\*\*] and [\*\*]. uniQure will make available for BMS' use in connection with same, subject to Applicable Law and the terms under which same were provided to uniQure, any patient samples and related clinical data and other materials possessed and Controlled by uniQure.

## 5. COMMERCIALIZATION

**5.1 Commercialization of Products.** Except for uniQure's activities in connection with subcommittees and working groups established by the JSC, as between BMS and uniQure, BMS shall have the sole right and responsibility for the Commercialization of Products in the Field in the Territory at its cost and expense. BMS itself and/or through its Affiliates and Sublicensees will use Diligent Efforts to Commercialize each Product in each Major Market in which Regulatory Approval has been obtained for such Product. For the avoidance of doubt, the Parties assume that any activities of commercial exploitation hereunder will only occur [\*\*] and, should any such commercial exploitation activities occur at [\*\*], such [\*\*].

**5.2 The Commercial Plan.** The Commercialization of Products in the Field by BMS, including its Affiliates and Sublicensees, shall be conducted pursuant to a plan that outlines the key activities to be undertaken by BMS to Commercialize Products in the Major Markets (the "**Commercial Plan**"). The Commercial Plan shall provide an overview of key activities and the projected timelines for completion thereof projected for the Commercialization of the applicable Product in the Field in the Major Markets and

does not have to be any more detailed than the commercial plans BMS prepares for its own commercialized therapeutic products. The initial Commercial Plan (the "**Initial Commercial Plan**") shall be provided by BMS to JSC at least [\*\*] months prior to the First Commercial Sale of a Product in the Territory for review and comment. For the avoidance of doubt, the Initial Commercial Plan is part of the Commercial Plan.

**5.3 Updates to the Commercial Plan.** Following BMS' submission of the Initial Commercial Plan, thereafter [\*\*] until [\*\*] years after [\*\*], BMS shall provide uniQure through the JSC with annual updates to the Commercial Plan. In the event [\*\*] (e.g. [\*\*]), [\*\*], through its Alliance Manager, will promptly notify [\*\*]. In the event [\*\*]. For the avoidance of doubt, any such material change shall not itself constitute a breach of BMS' obligation under Section 5.1 to use Diligent Efforts. For further avoidance of doubt, the fact that BMS informs uniQure about any material change in accordance with this Section 5.3, shall be without any prejudice to uniQure's right to claim a breach of BMS' obligation under Section 5.1 to use Diligent Efforts pursuant to this Agreement, independent of whether or not such allegation of a breach of Diligent Efforts is based on the material change that uniQure has been informed about or on any other act or omission of BMS.

**5.4 Commercialization Report.** For each Calendar Year following Regulatory Approval for a Product in a Major Market, BMS shall provide to uniQure annually within [\*\*] days after the end of such Calendar Year a written report that summarizes the Commercialization activities on a Collaboration Target-by-Collaboration Target and Product-by-Product basis performed by BMS and its Affiliates and Sublicensees in the Major Markets since the prior report by BMS. Such reports shall be Confidential Information of BMS pursuant to Article 12.

**5.5 Commercial Forum.** BMS shall keep uniQure informed of BMS' Commercialization activities through meetings of the Commercial Forum. The Commercial Forum will [\*\*], with the [\*\*] determining the [\*\*] for the Commercial Forum.

**5.6 Decision-Making Authority.** As between BMS and uniQure, BMS shall have the sole decision-making authority for the operations and Commercialization strategies and decisions, including funding and resourcing, related to the Commercialization of Products in the Field in the Territory.

## **6. THERAPEUTIC SUPPLY AND MANUFACTURING**

**6.1 Therapeutic Supply.** uniQure shall be responsible for the production and supply to BMS of sufficient quantities of Therapeutics for the testing of such Therapeutics in the performance of the Research Program in accordance with the Research Plan. Upon request by BMS, uniQure shall transfer to BMS uniQure's inventory of Therapeutics prepared by or for uniQure in the conduct of the Research Program; *provided however*, that uniQure shall retain that portion of such inventory required by uniQure to fulfill its responsibilities under the Research Plan. The costs of uniQure for the production and supply to BMS of such Therapeutics of non-GMP grade shall be compensated by the FTE based funding pursuant to Section 3.5. The production and supply to BMS of clinical and commercial GMP grade Therapeutics and Products shall be governed by the Supply Agreement and the statements of work under the Supply Agreement.

**6.2 Manufacturing Overview.** Within the scope of the licenses granted in Section 7.1(a) and (b) and subject to the payment obligations of BMS pursuant to Article 8 and the provisions set forth in Section 6.3 below, BMS shall have the exclusive right and will be solely responsible for the manufacture of Therapeutics that are not for Gene Therapy (e.g., peptides) and Products containing such Therapeutics ("**Non-Gene Therapy Therapeutics**" and "**Non-Gene Therapy Products**", respectively) itself or through one or more Affiliates or Third Parties selected by BMS. For all Therapeutics and Products that are for Gene Therapy, uniQure shall be responsible for the clinical and commercial manufacture and supply of such Therapeutics and Products, and the Parties (a) will enter into a supply agreement for such Therapeutics and Products (the "**Supply Agreement**") within [\*\*] months of the Effective Date, (b) shall [\*\*] for the clinical supply of each [\*\*] to be administered [\*\*] within [\*\*] months after [\*\*], and (c) shall [\*\*] for the

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commercial supply of each [\*\*] within [\*\*] months after BMS designates such Therapeutic to move into full development. The Supply Agreement shall contain the provisions set forth in **Exhibit J**, and the commercial drug product responsibilities will be set forth in the Supply Agreement.

**6.3 Third Party Manufacturing.** Within the scope of the licenses granted in Section 7.1(a) and (b) and subject to the payment obligations of BMS pursuant to Article 8, BMS may exercise any of its manufacturing rights with respect to Non-Gene Therapy Therapeutics and Non-Gene Therapy Products through one or more Third Party manufacturers; *provided however*, that the Third Party manufacturer undertakes in writing obligations of confidentiality and non-use regarding Confidential Information of uniQure (including uniQure Manufacturing Technology received by such Third Party manufacturer) that are consistent with those undertaken by the Parties pursuant to Article 12 hereof; *provided further*, that the term of such Third Party's obligations regarding the use and disclosure of Confidential Information and Know-How shall be as long as can be reasonably negotiated with such Third Party, but in any event no less than [\*\*] years after the date of disclosure to the Third Party. Such Third Party manufacturers shall be obligated in writing not to use the uniQure Know-How and uniQure Manufacturing Technology for any use, other than the manufacture of Non-Gene Therapy Therapeutics and Non-Gene Therapy Products for BMS, its Affiliates and Sublicensees.

## **7. GRANT OF RIGHTS AND LICENSES**

### **7.1 License to BMS.**

(a) **Exclusive License Grant.** Subject to the terms and conditions of this Agreement and the terms and conditions of any Third Party agreement that are applicable to a sublicensee under such Third Party agreement (including the Existing License Agreements), uniQure hereby grants to BMS an exclusive (even as to uniQure, except as provided in Section 7.3) license, with the right to grant sublicenses (through multiple tiers) as provided in Section 7.2, under the uniQure Technology to research, develop, make, have made, use, sell, offer for sale, export and import (including the exclusive right to Develop and Commercialize) Collaboration Targets, Therapeutics and Products in the Field in the Territory. Without limiting the generality of the foregoing terms of this Section 7.1(a), the license granted by uniQure to BMS pursuant to this Section 7.1(a) shall include, subject to the terms and conditions of this Agreement and the terms and conditions of any Third Party agreement that are applicable to a sublicensee under such Third Party agreement (including the Existing License Agreements), an exclusive (even as to uniQure, except as provided in Section 7.3) sublicense, with the right to grant sublicenses (through multiple tiers) as provided in Section 7.2, under the Information and Patents included in the uniQure Technology and licensed to uniQure under any Third Party Agreements to which uniQure is a party, to research, develop, make, have made, use, sell, offer for sale, export and import (including the exclusive right to Develop and Commercialize) Collaboration Targets, Therapeutics and Products in the Field in the Territory.

(b) **Non-exclusive License Grant.** Subject to the terms and conditions of this Agreement and the terms and conditions of any Third Party agreement that are applicable to a sublicensee under such Third Party agreement (including the Existing License Agreements), and without limiting the exclusive licenses granted by uniQure to BMS under Section 7.1(a), uniQure hereby further grants to BMS a non-exclusive license, with the right to grant sublicenses (through multiple tiers) as provided in Section 7.2, under the uniQure Technology to make any starting materials, intermediates and Raw Materials solely for use in making Therapeutics and Products in the exercise by BMS of the exclusive license granted to BMS under Section 7.1(a).

(c) **Non-exclusive Research Rights.** In addition, and subject to the terms and conditions of this Agreement and the terms and conditions of any Third Party agreement that are applicable to a sublicensee under such Third Party agreement (including the Existing License Agreements), and without limiting the exclusive and non-exclusive licenses granted by uniQure to BMS under Section 7.1(a) and (b),

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BMS shall have the non-exclusive right to use the Therapeutics and uniQure Know-How relating to the Therapeutics that are provided by or disclosed to BMS by uniQure during the Research Term for non-clinical research purposes in support of BMS' research programs relating to the biology of Collaboration Targets. In the exercise of such right, BMS shall have the right to transfer Therapeutics to a Third Party academic or other non-profit research institution collaborator (any such Third Party being a "**Collaborator**"); *provided however*, that:

(i) any such Collaborator shall be bound by obligations with respect to the use and disclosure of uniQure Confidential Information to the same extent applicable to BMS under Article 12;

(ii) any such Therapeutic will be used by the Collaborator solely for the research purposes of BMS relating to the applicable Collaboration Target in conducting research for the benefit of BMS, and shall not be used for any other purpose; and

(iii) BMS shall obtain the prior written consent of uniQure, such consent not to be unreasonably withheld, conditioned or delayed (in particular if the grant to or exercise of such right by the Collaborator will result in any payment obligation of uniQure or its Affiliates under the terms and conditions of any Third Party agreement that are applicable to a sublicensee under such Third Party agreement (including the Existing License Agreements)).

(d) **Existing License Agreements.** Notwithstanding Section 7.1(a), as of the Effective Date, the Parties agree and acknowledge that no sublicense is granted in this Agreement to BMS under any of the Information and Patents included in the uniQure Technology and licensed to uniQure under any of the Existing License Agreements. However, a sublicense to the Information and Patents included in the uniQure Technology and licensed to uniQure under a particular Existing License Agreements may be granted to BMS under this Agreement upon mutual agreement of the Parties or as provided in the Supply Agreement.

(e) **Limitations.** For clarity, BMS' licenses under this Section 7.1 shall cover only [\*\*] with respect to the applicable [\*\*], and only for so long as [\*\*]. Accordingly, the licenses under this Section 7.1 with respect to a particular [\*\*] with respect to [\*\*], shall [\*\*] when such [\*\*] and is therefore no longer a [\*\*].

**7.2 Sublicensing by BMS.** BMS shall have the right to sublicense any or all of the rights granted to it by uniQure under this Agreement to its Affiliates or to Third Parties without the consent of uniQure; *provided however*, BMS shall obtain the prior written consent of uniQure, such consent not to be unreasonably withheld, conditioned or delayed, if BMS wishes to grant any sublicense to any or all of the rights granted to uniQure under any Third Party agreement and such Third Party agreement requires the consent of the Third Party licensor before BMS can grant any sublicense or if the grant to or exercise of such right to sublicense will result in any sublicensing-specific (i.e., resulting from the grant or exercise of such sublicense) payment obligations of uniQure or its Affiliates other than the payment of royalties and/or milestones under the terms and conditions of such Third Party agreement (including any Existing License Agreement), uniQure shall not withhold, condition or delay its consent to BMS sublicensing such rights, if BMS agrees to be responsible for all such sublicensing-specific payment obligations of uniQure or its Affiliates under the terms and conditions of any such Third Party agreement (including any Existing License Agreement). **Exhibit L** contains a list of such Third Party agreements as of the Effective Date and with respect to each such Third Party agreement, whether such Third Party agreement requires the consent of the Third Party licensor before BMS may grant any sublicense and/or if the grant to or exercise of such right to sublicense will result in any sublicensing-specific payment obligation of uniQure or its Affiliates other than the payment of royalties and/or milestones under the terms and conditions of such Third Party agreement. uniQure shall update Exhibit L from time to time by providing written notice to BMS to add Third Party agreements that require such Third Party licensor consent and/or result in any such sublicensing-specific payment. If a Third Party agreement is not listed in Exhibit L, then BMS shall have no obligation to seek the prior written consent of uniQure to sublicense any or all of the rights granted to it by uniQure under such

Third Party agreement. In connection with any such sublicensing and to the extent required to carry out the applicable sublicensing, BMS may disclose and provide to such permitted Sublicensees any applicable uniQure Know-How and uniQure Materials in connection therewith. BMS shall ensure that each of its Third Party Sublicensees (or each of its Sublicensees if so required under any Third Party agreements) is bound by a written agreement that is consistent with, and subject to the applicable terms and conditions of, this Agreement. In addition, BMS shall be responsible for the performance of any of its Sublicensees that are exercising rights under a sublicense of the rights granted by uniQure to BMS under this Agreement, and the grant of any such sublicense shall not relieve BMS of its obligations under this Agreement, except to the extent they are satisfactorily performed by any such Sublicensee(s). Promptly following the execution of each Third Party sublicense agreement (or each sublicense agreement if so required under any Third Party agreements) as provided in this Section 7.2, BMS shall provide uniQure with a copy of each such sublicense agreement; *provided however*, financial terms may be redacted to the extent permitted by the applicable Third Party agreement with uniQure (including the applicable Existing License Agreement). Any Sublicensee (including an Affiliate of BMS) shall have the right to grant further sublicenses to its Affiliates or Third Parties of the same or lesser scope as its sublicense obtained from BMS, any Affiliate of BMS or any other Sublicensee under this Section 7.2 (such further sublicensee to such further sublicense also being a Sublicensee); *provided however*, that such further sublicenses shall be in accordance with and subject to all of the terms and conditions of this Section 7.2 (i.e., such Sublicensee shall be subject to this Section 7.2 in the same manner and to the same extent as BMS, including obtaining the prior written consent of uniQure under the same circumstances that BMS would be required to obtain such prior written consent of uniQure).

### **7.3 Limited Grant Back to uniQure.**

(a) Subject to the terms and conditions of this Agreement, BMS hereby grants to uniQure and its Affiliates a non-exclusive, non-sublicensable (except to subcontractors permitted under Section 3.11) royalty-free license under the uniQure Technology licensed pursuant to Section 7.1 (including the Product Specific Patents), the Joint Inventions, the Joint Patents, the BMS Patents and any other Information and Patents Controlled by BMS or its Affiliates during the Term that in each case is necessary for uniQure and its Affiliates to conduct their Research Program activities and perform their other obligations under this Agreement and the Supply Agreement, and such license is granted solely for uniQure and its Affiliates to conduct their Research Program activities and perform their other obligations under this Agreement and the Supply Agreement.

(b) Subject to the terms and conditions of this Agreement, BMS hereby grants to uniQure and its Affiliates a non-exclusive, fully paid up, royalty-free, irrevocable, perpetual and unlimited (i.e., during the Term and thereafter) license, with the right to assign and sublicense, under the uniQure Technology licensed pursuant to Section 7.1, the Joint Inventions, the Joint Patents and the BMS Patents to use and exploit (including to commercially use and exploit) the uniQure Platform Technology and uniQure Platform Technology Improvements in any manner consistent with the licenses granted by uniQure to BMS under Section 7.1 and the exclusivity undertaking in Article 11.

**7.4 No Other Rights.** Except for the rights expressly granted under this Agreement, no right, title, or interest of any nature whatsoever is granted whether by implication, estoppel, reliance, or otherwise, by a Party to the other Party. All rights with respect to Information, Patent or other intellectual property rights that are not specifically granted herein are reserved to the owner thereof.



**7.5 Public Domain Information.** Nothing in this Agreement shall prevent either Party or its Affiliates from using for any purpose any Know-How that is in the public domain or Confidential Information that is no longer protected by confidentiality under Article 12.

**7.6 Liens.**

- (a) Except as disclosed in **Exhibit R** or to the extent permitted under Section 17.8,

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uniQure shall not during the Term grant any Lien (or permit any Lien to attach) with respect to the uniQure Technology licensed pursuant to Section 7.1 (including any of the Product Specific Patents) that would adversely impact BMS' rights under this Agreement. Any breach of this Section 7.6 (a) by uniQure shall be deemed a material breach of this Agreement.

- (b) With respect to the loan agreements which govern the liens listed in Exhibit R, uniQure shall [\*\*] every [\*\*] months and upon request of BMS. In case that uniQure [\*\*], uniQure shall notify BMS thereof and in such case BMS shall [\*\*]. If and to the extent that [\*\*], [\*\*].

**7.7 Compliance with Third Party Licenses.**

(a) The license grant by uniQure under the uniQure Technology set forth in Section 7.1 includes the grant of a sublicense to BMS of certain uniQure Technology that is not owned by uniQure or its Affiliates (including the grant of a sublicense under the Existing License Agreements). BMS' rights and licenses under, or with respect to, the uniQure Technology (including the Prosecution or Enforcement of any Patents undertaken by the Parties pursuant to Article 9) are limited to the rights granted by such Third Party licensors (including Existing Third Party Licensors) to uniQure under any license agreements with such Third Parties (including the Existing License Agreements) (the "**Third Party Licenses**") and are subject to all applicable restrictions, limitations, obligations and reservations (including for the benefit of such Third Party licensors or the U.S. Government) imposed on uniQure or its sublicensees in such Third Party Licenses. BMS shall comply, and cause its Affiliates and Sublicensees to comply, with all such restrictions, limitations and obligations. To the extent there is a conflict between the terms of any Third Party License and the rights granted to BMS hereunder, the terms of such Third Party License shall control solely with respect to the Patent rights and know-how owned or controlled by the applicable Third Party licensor. Notwithstanding anything to the contrary in this Agreement, either Party may not exercise any of its rights or delay performance of any of its obligations under this Agreement in any manner that would result in any licensor having a right to terminate a Third Party License, or that would cause the other Party to be in breach of any of its obligations under any Third Party License.

(b) During the Term, uniQure shall comply with the Third Party Licenses in effect which are then applicable to the activities under this Agreement and/or the Supply Agreement (and in particular shall not commit any breach that would entitle the Third Party licensor to terminate such a Third Party License) and shall not terminate any such Third Party License without BMS' prior written consent. Without limiting any other right or remedy of BMS under this Agreement and in order to prevent, ameliorate, mitigate or cure a breach of any of the Third Party Licenses, in the event that uniQure becomes aware (either on its own, or by notice from its licensor) of its material failure to perform any of its obligations under any of such Third Party Licenses (including where a breach or alleged breach by BMS of its obligations under this Agreement or any other act or omission by BMS prevents such performance by uniQure or is the cause of uniQure's failure to perform such obligation), it shall so notify BMS in writing, and if such failure is not cured within thirty (30) days after written notice to BMS, BMS shall have the right to perform such obligation on behalf of uniQure. Except to the extent that a breach or alleged breach by BMS of its obligations under this Agreement or any other act or omission by BMS prevents such performance by uniQure or is the cause of uniQure's failure to perform such obligation, uniQure shall reimburse BMS for its costs and expenses (excluding, however, internal costs) in connection with such performance. To the extent the Parties dispute whether any breach, alleged breach or other act or omission by BMS prevented such performance or was the cause of uniQure's failure to perform such obligation, the Parties shall resolve such disputes pursuant to Section 9.14 or Article 16, as applicable. This Agreement sets forth the obligations of the Parties *inter se*, and nothing in this Agreement (including any standard of effort set forth herein) shall limit or modify the obligations of uniQure under the Third Party Licenses.

(c) Any sublicensee obligations required by any Third Party License to be included in a sublicense thereunder, including any required provision making the applicable Third Party licensor a third

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party beneficiary of any sublicense thereunder, shall be deemed to be included in this Agreement.

(d) To the extent that uniQure is permitted to assert against a Third Party licensor of a Third Party License (including an Existing Third Party Licensor) a claim on behalf of BMS (as uniQure's sublicensee) for specific performance of any covenant of a Third Party licensor contained in the applicable Third Party License, uniQure shall use reasonable efforts to cooperate with BMS (at BMS' expense) to permit BMS to assert such claim or request for specific performance by such Third Party licensor, including, if necessary, allowing BMS to bring such claim in the name of uniQure; *provided however*, that BMS shall give uniQure written notice of any proposed settlement with such Third Party licensor and a reasonable opportunity to review and comment on such proposed settlement, and BMS shall not enter into any settlement with such Third Party licensor that could reasonably be viewed as adversely affecting the rights of uniQure hereunder or under the applicable Third Party License or otherwise adversely affecting uniQure, without uniQure's prior written consent (such consent not to be unreasonably withheld, delayed or conditioned).

(e) Whenever uniQure provides any report, notice or other communication to a Third Party licensor of a Third Party License relating to Therapeutics, Products and/or this Agreement (or otherwise relating to or impacting the rights sublicensed to BMS under this Agreement) in compliance with any of the obligations under the Third Party Licenses, uniQure shall provide a copy of such report or notice to BMS at least ten (10) days prior to the time such report, notice or communication is provided to such Third Party licensor or, if it is impracticable for uniQure to provide such copy at least ten (10) days ahead of time, uniQure shall provide such copy to BMS as early as practicable prior to the provision thereof to such Third Party licensor.

(f) Whenever uniQure receives any report, notice or other communication relating to Therapeutics, Products and/or this Agreement (or otherwise relating to or impacting the rights sublicensed to BMS under this Agreement) from a Third Party licensor with respect to the applicable Third Party License (including any notice with respect to any default, breach or termination of the Third Party License), uniQure shall promptly provide a copy of such report, notice or other communication to BMS.

(g) uniQure shall, if reasonably requested by BMS and to the extent uniQure reasonably agrees with BMS' interpretation of such rights, take commercially reasonable efforts to exercise any of uniQure's rights, or to enforce any material obligation of a Third Party licensor, under the applicable Third Party License, in each case as it relates to a Collaboration Target, Therapeutic and/or Product.

(i) uniQure shall not agree or consent to or enter into any amendment, supplement or other modification to the Third Party Licenses, or exercise any other right or consent thereunder, in each case in a manner that could reasonably be viewed at such time as adversely affecting the Patent and/or other intellectual property rights granted to uniQure under such Third Party Licenses and sublicensed to BMS under this Agreement, without BMS' prior written consent (such consent not to be unreasonably withheld, delayed or conditioned).

(ii) uniQure shall not terminate, and shall not take or fail to take any action that would permit any Third Party licensor to terminate, any Third Party License (either unilaterally or by mutual agreement with the applicable Third Party licensor), or any right thereunder, without the prior written consent of BMS (such consent not to be unreasonably withheld, delayed or conditioned), in each case as it relates to or impacts the Patent and/or other intellectual property rights granted to uniQure under such Third Party License and sublicensed to BMS under this Agreement.

## 7.8 Additional Rights Acquired after Effective Date.

(a) During the Term, if either Party identifies the need for, or is otherwise offered, a license, covenant not to sue or similar rights to any Third Party Patent rights or Third Party know-how that

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such Party in good faith believes is necessary or reasonably useful for the research, Development or Commercialization of Therapeutics or Products in the Field in the Territory (the "**Additional Rights**"), then such Party shall promptly and, in any event, prior to commencing negotiations or entering into an agreement with respect to such Additional Rights, notify the other Party, and the Parties' rights to conduct such negotiations shall be subject to the remaining provisions of this Section 7.8. The Parties shall thereafter conduct good faith discussions regarding whether such Additional Rights are necessary or reasonably useful for the research, Development or Commercialization of Therapeutics or Products in the Field in the Territory or whether they otherwise agree that such Additional Rights should be acquired.

(b) uniQure shall have the first right (but not the obligation) to license or otherwise acquire rights to any Additional Rights that have general application to the manufacture of AAV based products or that, if licensed to or otherwise acquired by uniQure, would fall within the definition of uniQure Platform Technology. BMS shall have the first right (but not the obligation) to license or otherwise acquire rights to all other Additional Rights. If the applicable Party provides written notice to the other Party that it declines to exercise such first right, then the other Party shall have the right (but not the obligation) to pursue acquiring rights to any given Additional Rights. The Party pursuing any given Additional Rights (the "**Controlling Party**") shall confer with the other Party (the "**Non-Controlling Party**") about the approach to take and keep the Non-Controlling Party reasonably informed regarding the status thereof and shall use Diligent Efforts to obtain from the applicable Third Party licensor the right to sublicense such Additional Rights under the licenses granted to the Non-Controlling Party hereunder. Notwithstanding anything to the contrary in this Section 7.8, the Non-Controlling Party shall have the right to obtain rights to any Additional Rights if the Parties cannot agree on the approach to be taken by the Controlling Party in securing rights to the Additional Rights for the Non-Controlling Party or if the Non-Controlling Party determines in good faith that it is necessary to do so (e.g., where the Non-Controlling Party needs a license to the Additional Rights before the Controlling Party does), in which case the Non-Controlling Party shall only obtain rights to any such Additional Rights that serve the purposes of the Non-Controlling Party under this Agreement.

(c) If the Controlling Party acquires rights to any Additional Rights and has the right to grant a sublicense under such Additional Rights to the Non-Controlling Party, and the Non-Controlling Party wishes to include such Additional Rights in the licenses granted to the Non-Controlling Party hereunder, the Non-Controlling Party shall notify the Controlling Party of its desire to do so and the Controlling Party shall provide the Non-Controlling Party a summary of all material restrictions on the scope of the licenses granted under, and all material payment obligations that would be owed by the Non-Controlling Party with respect to, any Third Party agreement applicable to such Additional Rights. The Non-Controlling Party may, upon written notice to the Controlling Party and subject to Section 7.8(d), Section 7.8(e) and Section 7.8(f), obtain a sublicense under such Additional Rights and include such Additional Rights under the licenses granted to the Non-Controlling Party hereunder.

(d) Following such notice from the Non-Controlling Party that it desires to include any given Additional Rights under the license granted to the Non-Controlling Party hereunder, (i) any such Additional Rights that do not carry financial or other obligations or restrictions shall be included automatically under the applicable license hereunder, and (ii) subject to Section 7.8(e), any such Additional Rights that carry financial or other obligations or restrictions shall be included under the applicable license hereunder only if the Non-Controlling Party agrees to share the costs of such Additional Rights (including any upfront payment or similar acquisition cost to access such Additional Rights) with the Controlling Party and to assume all other obligations to, and be subject to all restrictions imposed by, the Controlling Party's licensor to the extent arising from the grant to the Non-Controlling Party under such Additional Rights (including, to the extent access to such terms have been made available to the Non-Controlling Party in unredacted form, all other terms of the Additional Rights Agreement that apply to the licenses granted to the Non-Controlling Party hereunder).

(e) If the Parties are unable, after twenty (20) Business Days, to agree as to whether any

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given Additional Rights are in fact necessary or reasonably useful for the research, Development or Commercialization of Therapeutics or Products in the Field in the Territory or if the Parties are unable to agree to the allocation of the costs (as specified above), then the Parties shall jointly engage an Expert (who is a patent attorney with the applicable expertise and experience in pharmaceutical patent law), appointed by uniQure and reasonably acceptable to BMS to resolve such dispute. The decision of such Expert shall be final and the costs of such decision shall be borne between the Parties in such manner as such Expert shall determine. For clarity, any dispute subject to dispute resolution pursuant to this Section 7.8(e) shall not be subject to the terms and conditions of Sections 16.2 to 16.5. If the Parties are unable to mutually agree on an Expert within the aforementioned twenty (20) Business Day period, the dispute shall be subject to the terms and conditions of Article 16.

(f) Nothing in this Section 7.8 shall restrict either Party, at such Party's sole cost and expense, from licensing or otherwise acquiring any additional rights that are not necessary or reasonably useful for the research, Development or Commercialization of Therapeutics or Product in the Field

in the Territory.

**7.9 Restriction on Transfer of Target Therapeutics to Third Parties by uniQure.** For the avoidance of doubt, and subject to Section 17.8, during the Term so long as a Target is a Collaboration Target under this Agreement, uniQure shall not transfer rights to any Target Therapeutic for such Collaboration Target or any Variant thereof in the Territory in the Field to any Third Party without the prior written consent of BMS. In addition, during the Research Term, so long as a Target is a Reserved Target, uniQure shall not transfer rights to any Target Therapeutic for such Reserved Target or any Variant thereof in the Territory in the Field to any Third Party without the prior written consent of BMS.

#### **7.10 Ex-US uniQure Patents.**

(a) **Security Rights.** In order to secure BMS' interest in the licenses granted to BMS in relation to uniQure Patents under this Agreement in case of an Insolvency Event of uniQure and/or any of its Affiliates Controlling uniQure Patents, uniQure shall, upon request and at the sole cost and expense of BMS, either (i) register or cause to register such licenses in any patent registry of the country where such patent has been granted and such registration can be effected outside the United States requested by BMS or (ii) grant to BMS one or more usufruct, security or any other related rights in addition to and in the same scope as the licenses granted under this Agreement, however solely for countries other than the United States. If above parts (i) or (ii) are ineffective or insufficient in BMS' patent counsel's reasonable opinion to fully secure BMS' interests in the licenses granted to BMS under this Agreement, and subject to Sections 7.10 (c), (d) and (e) and 13.9, uniQure and/or any of its Affiliates Controlling uniQure Patents agree to assign, and hereby do assign, one-half (1/2) of their right, title and interest in and to each Product Specific Patent outside of the U.S. that is not a Joint Patent ("**Ex-US Product Specific Patent**") to BMS so that, after such assignment, all Ex-US Product Specific Patents shall be jointly owned by uniQure and BMS. The security rights outlined in (i) and (ii) shall be granted by uniQure at the sole discretion of BMS, but solely alternatively, not cumulatively.

(b) **Release of Security Rights.** The Parties agree and acknowledge that the above BMS security rights in Section 7.10(a) are intended to survive and remain valid in the event that uniQure and/or any of its Affiliates Controlling uniQure Patents incurs an Insolvency Event. Notwithstanding, a particular security right shall be revoked and terminated simultaneously with a termination of the corresponding underlying uniQure Patent license right (either in general or on a country-by-country basis as the case may be pursuant to this Agreement) pursuant to Section 13.2 or Section 13.3(e). In order to secure uniQure's interest in a timely release of the uniQure Patents from the security rights following a termination of a license granted by uniQure to BMS under Section 13.3(e) of this Agreement, (i) BMS shall issue all declarations required for a future release of each uniQure Patent simultaneously with the creation of the respective security right and (ii) the Parties shall mutually agree on a trustee who shall hold such

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documentation in trust for both Parties and who shall hand over such documentation to uniQure solely in accordance with the conditions reflecting the termination provisions in this Agreement to be outlined in a respective trustee agreement mutually agreed by the Parties and such trustee and (iii) shall transfer such documentation to such trustee without undue delay.

(c) With respect to any Ex-US Product Specific Patents existing as of the Effective Date, BMS may provide to uniQure (and/or its relevant Affiliates) and uniQure (and/or its relevant Affiliates) shall execute and deliver to BMS, at BMS' expense, mutually agreed upon documents in the forms required in the applicable jurisdictions in order to perfect the assignment to BMS of the one-half (1/2) interest in and to the Ex-US Product Specific Patents. For any Patents that become Ex-US Product Specific Patents after the Effective Date (i.e., by virtue of being filed after the Effective Date), uniQure (and/or its relevant Affiliates) shall assign, and hereby does assign effective as of the date that such Patent becomes an Ex-US Product Specific Patent, one half (1/2) of its right, title and interest in and to each such Patents to BMS within ninety (90) days after such Patents are deemed to have become Ex-US Product Specific Patents. BMS may provide to uniQure (and/or its relevant Affiliates) and uniQure (and/or its relevant Affiliates) shall execute and deliver to BMS, at BMS' expense, mutually agreed upon documents in the forms required in the applicable jurisdictions in order to record or perfect the assignment to BMS of the one-half (1/2) interest in and to any post-Effective Date Ex-US Product Specific Patents. BMS shall be responsible for recording all such assignments and uniQure (and/or its relevant Affiliates) shall reasonably cooperate, at BMS' expense, with BMS' efforts to do so. Notwithstanding such assignment, the Ex-US Product Specific Patents shall remain uniQure Patent Rights.

(d) The assignment of Ex-US Product Specific Patents to BMS pursuant to Section 7.10(a) shall in no way alter BMS' Net Sales Compensation obligations to uniQure under this Agreement with respect to such Ex-US Product Specific Patents as set forth in this Agreement. An Ex-US Product Specific Patent shall not become a Joint Patent or a BMS Patent by reason of the assignment contemplated by this Section 7.10, and shall at all times remain a uniQure Patent Right that is a Product Specific Patent. In addition, (i) uniQure (and/or its relevant Affiliates) shall have the right to exploit, license and grant a security interest in (in all cases, subject to and without limiting the terms and conditions of this Agreement, including uniQure's obligations and the rights and licenses granted to BMS under this Agreement, to the extent then in effect) the Ex-US Product Specific Patents without the consent of or a duty of accounting to BMS; (ii) BMS shall not practice the Ex-US Product Specific Patents outside the scope of the licenses granted to BMS in Article 7; (iii) BMS shall have the right to grant licenses under the Ex-US Product Specific Patents only in accordance with its sublicensing rights under Section 7.2, and BMS shall not encumber the Ex-US Product Specific Patents; and (iv) BMS shall not have any rights with respect to the Ex-US Product Specific Patents beyond the scope of the rights conferred pursuant to the license granted in Section 7.1.

(e) For any Patents that are not Joint Patents that cease to be Ex-US Product Specific Patents at any time during the Term by virtue of an amendment of the claims (or following replacement or termination of the applicable Collaboration Target or termination under Article 13 of rights with respect to the applicable Ex-US Product Specific Patent), BMS shall assign, and hereby does assign effective as of the date that uniQure notifies BMS in writing that such Patent is no longer a Ex-US Product Specific Patent, its entire right, title and interest in and to each such Patent to uniQure or uniQure's designee, and BMS appoints, effective as of such date, uniQure as its attorney in fact solely to make such re-assignments and authorizes uniQure to make such re-assignments. In each case, BMS shall execute and deliver to uniQure a deed(s) of such assignment, in a mutually agreeable form, within thirty (30) days after the date such Patent ceased to be a Ex-US Product Specific Patent. uniQure shall be responsible for recording all such assignments and BMS and its successors and assigns shall (i) reasonably cooperate with uniQure's efforts to do so, including satisfying the assignment and recording requirements of relevant patent offices and (ii) reimburse uniQure for all expenses incurred by uniQure in connection with this Section 7.10(e). In addition, BMS hereby grants uniQure an exclusive, royalty-free, fully sublicensable license under its interest in each such former Ex-US

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Product Specific Patent during the period from the date such Patent ceased to be an Ex-US Product Specific Patent until such former Ex-US Product Specific Patent is actually re assigned to uniQure or uniQure’s designee.

8. PAYMENTS

8.1 Upfront Payment. BMS shall pay uniQure an upfront payment of fifty million Dollars (\$50,000,000) within fifteen (15) Business Days after the Effective Date.

8.2 Equity Investment. As of the Signing Date, and in conjunction with this Agreement, BMS and uniQure’s Affiliate, uniQure N.V., have entered into the Investor Agreement, the Share Subscription Agreement, the Seventh Collaboration Warrant Agreement and the Tenth Collaboration Warrant Agreement (collectively with this Agreement, the “Transaction Agreements”) regarding the acquisition by BMS of ordinary shares, par value €0.05 per share, of uniQure N.V. (“Ordinary Shares”).

8.3 Development Milestone Payments for Product.

(a) Milestones. BMS shall further pay to uniQure the milestone payments as described below for each Therapeutic within [\*\*] days after the first achievement of the specified milestone event by BMS, its Affiliates or their Sublicensees for each Therapeutic. BMS shall provide written notice to uniQure within [\*\*] Business Days after the achievement of each specified milestone event for each Therapeutic by BMS or its Affiliates and within [\*\*] days after each achievement of the specified milestone event by its Sublicensees or their Affiliates for each Therapeutic.

Development Milestone Event	Milestone Payments					
	Lead S100A1 Therapeutics and a Lead S100A1 Back-up Therapeutic in the event it replaces a Lead S100A1 Therapeutic				Therapeutics For All Other Collaboration Targets, including Back-up Therapeutics (except a Lead S100A1 Back-up Therapeutic in the event it replaces a Lead S100A1 Therapeutic)	
	First Distinct Indication	Second Distinct Indication	First Distinct Indication	Second Distinct Indication	First Distinct Indication	Second Distinct Indication
[**]	[**]	[**]	[**]	[**]	[**]	[**]
[**]	[**]	[**]	[**]	[**]	[**]	[**]
[**]	[**]	[**]	[**]	[**]	[**]	[**]
[**]	[**]	[**]	[**]	[**]	[**]	[**]
[**]	[**]	[**]	[**]	[**]	[**]	[**]
[**]	[**]	[**]	[**]	[**]	[**]	[**]
[**]	[**]	[**]	[**]	[**]	[**]	[**]
[**]	[**]	[**]	[**]	[**]	[**]	[**]
[**]	[**]	[**]	[**]	[**]	[**]	[**]

[**]	[**]	[**]	[**]	[**]
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- (b) Interpretation. For the avoidance of doubt:
- (i) “Initiation” as used in the table in Section 8.3(a) means [\*\*].
  - (ii) For a given Therapeutic only one [\*\*] as set forth in the table in Section 8.3(a) upon the [\*\*].
  - (iii) Milestone payments for a “[\*\*]” milestone event must be [\*\*].
  - (iv) If [\*\*], MAA Filing shall be [\*\*].
  - (v) BLA Approval, MAA Approval and JNDA Approval [\*\*].
  - (vi) If Development is discontinued for a Therapeutic for a Collaboration Target, then, upon the decision to discontinue such Therapeutic, the following [\*\*] shall apply: (aa) [\*\*] shall remain valid and applicable and (bb) [\*\*], if any, shall remain valid and applicable, whereas (cc) [\*\*] shall not be applicable and payable, if such [\*\*] and (dd) all milestone payments triggered thereafter for the subsequent Therapeutic or Back-up Therapeutic shall [\*\*]. [\*\*].
  - (vii) Where a Phase 1 Clinical Trial for a Therapeutic or Product is designed to include an expansion phase to a Phase 2 Clinical Trial for such Therapeutic or Product, then [\*\*] will be deemed to have been achieved when [\*\*], and BMS will [\*\*].
  - (viii) Where, following the completion of a Phase 2 Clinical Trial for a Therapeutic or Product, such Phase 2 Clinical Trial is deemed a registrational Clinical Trial such that a Phase 3 Clinical Trial for such Therapeutic or Product is not conducted, then upon the earlier of [\*\*], the [\*\*] milestone event for such Therapeutic or Product shall [\*\*].

8.4 Sales Milestone Payments for Product.

(a) Milestones. For each Product, the following sales based milestone payments shall be payable by BMS based on the total level of annual Net Sales (i.e., Net Sales in a given Calendar Year period) being first reached by such Product in the Field in the Territory by BMS, its Affiliates and Sublicensees.

Sales Milestone	Milestone Payment
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(ii) Immediately upon signing of this Agreement, uniQure shall use its best efforts to procure a written agreement between uniQure, Prof. [\*\*] and Prof. [\*\*] in which (a) they waive their statutory publication rights pursuant to the German Law on Employee Inventions (*Arbeitnehmererfindergesetz*) and any other publication right under Applicable Law as it relates to any Information relating to the Lead S100A1 Therapeutics and/or the Research Program generated prior to or on or after the Effective Date, (b) they agree that with respect to any Information relating to the Lead S100A1 Therapeutics and/or the Research Program generated prior to the Effective Date, they shall only have the publication rights set forth in Exhibit M, and (c) they agree that with respect to any Information relating to the Lead S100A1 Therapeutics and/or the Research Program generated on or after the Effective Date shall be subject to Section 12.4.

(iii) Immediately upon signing of this Agreement uniQure shall use its best efforts to procure at its own expense [\*\*] in which [\*\*] as of the Signing Date that (1) [\*\*], and (2) [\*\*]; provided, however, such [\*\*] must be reviewed by and approved by [\*\*]. uniQure and/or uniQure GmbH shall be [\*\*].

(iv) Immediately upon signing of this Agreement, uniQure shall use its best efforts to [\*\*] in which (a) [\*\*], (b) [\*\*], and (c) [\*\*].

(v) Promptly after the Effective Date, [\*\*] shall initiate discussions with [\*\*] and shall attempt to negotiate a [\*\*] under which the Parties will provide [\*\*], each of [\*\*]. Until such [\*\*], uniQure shall not [\*\*]. In the event that [\*\*] to perform any research or Development activities for the Research Program, a [\*\*].

(vi) The obligation of uniQure pursuant to Section 8.5(c)(i) shall not include any [\*\*] for a license under [\*\*] Patents Covering [\*\*] to research, develop, make, have made, use, sell, offer for sale, export and import (including the right to Develop and Commercialize) Therapeutics and Products in the Field or in one or more [\*\*]. For any license agreement to any of [\*\*] Patents [\*\*], [\*\*] and would negotiate in good faith with [\*\*] the terms and conditions of any such agreement on terms mutually acceptable to [\*\*]; *provided however*, all upfront, milestone and other non-royalty payments will be [\*\*], and [\*\*], except that [\*\*]. Therefore, [\*\*]. In case that [\*\*] undertakes legal proceeding against [\*\*] with regard to a potential infringement of [\*\*] Patents and [\*\*] becomes obliged to acquire a license from [\*\*] on basis of a judicial decision or a settlement agreement, the Parties' agreement on cost sharing pursuant to this Section 8.5.(c)(iii) shall apply accordingly.

(vii) Subject to Section 8.5(c)(i), (ii), (iii) and (vi) and Section 8.5(g), [\*\*] obligations to uniQure under this Agreement shall be [\*\*] of the amount of the payments made by [\*\*] in accordance with Section 7.8 for the [\*\*], on account of [\*\*]. The foregoing shall not apply to [\*\*].

(d) **Generic Competition.** Subject to Section 8.5(g), during the portion of the applicable TC Term in a particular country of the Territory where there are one or more products being sold in such country that are Generic Products with respect to such Product, and the number of unit equivalents of

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such Generic Product(s) sold in such country in a particular Calendar Quarter equals or exceeds [\*\*] of the number of units of the applicable Product sold in such country before the introduction of the Generic Product(s) as measured by IMS or a mutually agreed Third Party, the tier percentages in Section 8.5(a) (ii) above used to calculate the Net Sales Compensation payable by BMS on Net Sales of such Product in such country shall be reduced by [\*\*]. In a country or jurisdiction where there is no Applicable Law to make a determination as to whether a product is a Generic Product, if the Parties are unable, after twenty (20) Business Days, to mutually agree on whether a product is or is not a Generic Product in such country or jurisdiction under Section 1.72 taking into consideration 42 USC 262(i) and Article 10, paragraph 2(b) of European Directive 2001/83/EC, then the Parties shall jointly engage an Expert (who is a regulatory attorney with the applicable expertise and experience in pharmaceutical regulatory law), appointed by uniQure and reasonably acceptable to BMS to resolve such dispute. The decision of such Expert shall be final and the costs of such decision shall be borne between the Parties in such manner as such Expert shall determine. For clarity, any dispute subject to dispute resolution pursuant to this Section 8.5(d) shall not be subject to the terms and conditions of Sections 16.2 to 16.5. If the Parties are unable to mutually agree on an Expert within the aforementioned twenty (20) Business Day period, the dispute shall be subject to the terms and conditions of Article 16.

(e) **TC Term.** Total Compensation payable by BMS to uniQure under Section 8.5 shall be paid on a Product-by-Product and country-by-country basis until the later of (i) [\*\*] years after the First Commercial Sale of the applicable Product in such country, (ii) [\*\*] in such country where the sale of the applicable Product in the applicable country would [\*\*], (iii) the [\*\*], or (iv) the [\*\*] (such period being the "**TC Term**" for a particular Product). Notwithstanding anything in this Section 8.5 to the contrary, and subject to Section 8.5(g), the tier percentages in Section 8.5(a)(ii) above used to calculate the Net Sales Compensation payable by BMS on Net Sales of such Product in such country shall be reduced by [\*\*] for the [\*\*]). For clarity, BMS shall [\*\*].

(f) **Application of Offset.** Any offsets to be applied pursuant to Sections 8.5(c)(vii), 8.5(d) and 8.5(e) shall be applied first to the highest tier of Net Sales Compensation being paid, and thereafter to successively lower tiers as needed to implement same.

(g) **Maximum Offset.** For the avoidance of doubt, the Net Sales Compensation payable under Section 8.5(a) taking into account any applicable offsets or reductions under Sections 8.5(c)(vii), 8.5(d) and 8.5(e) shall not be reduced in any such event below [\*\*] of the amount that would otherwise be due pursuant to Section 8.5(a) with respect to sales of a Product in a country in any Calendar Quarter. If, but for the preceding sentence, the deductions under Sections 8.5(c)(vii), 8.5(d) and 8.5(e) would have reduced a Net Sales Compensation payment made by BMS by more than [\*\*], then the amount of such deduction that exceeds [\*\*] will be carried over to subsequent Net Sales Compensation payments under Section 8.5 on sales of such Product in the Field anywhere in the Territory until the full amount that BMS would have been entitled to deduct (absent the above limitation) is deducted.

**8.6 Offset for Payments to Existing Third Party Licensors.** In the event that BMS is required to pay any royalties, milestones or other payments to any Existing Third Party Licensor (a) with respect to the research, Development or Commercialization of any Therapeutic or Product in the Field in the Territory that uniQure would otherwise be required to pay (but, although due, fails to pay in accordance with the applicable payment terms) under the corresponding Existing License Agreement, or (b) following the termination of the corresponding Existing License Agreement with the consent of uniQure in connection with BMS obtaining rights to uniQure Technology directly from the corresponding Existing Third Party Licensor that were sublicensed to BMS hereunder prior to such termination, then, notwithstanding anything in this Agreement to the contrary, [\*\*].

**8.7 Total Compensation Payments and Reports.** All amounts payable to uniQure pursuant to Section 8.5 shall be paid in Dollars within [\*\*] after the end of the Calendar Quarter in which the applicable

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Net Sales were recorded. To the extent any amounts payable to uniQure pursuant to Section 8.5(a)(i) (i.e., the Manufacturing Cost-Based Component of Supply Price) are actually paid to uniQure under the Supply Agreement, BMS may deduct such amounts from payment of Total Compensation under this Section 8.7. Each quarterly payment shall be accompanied by a Total Compensation report providing a statement, on a Product-by-Product and country-by-country basis, of: (a) the amount of Net Sales of Products in the Territory during the applicable Calendar Quarter, (b) a calculation of the amount of Total

Compensation in Dollars for the Products included in Net Sales for such Calendar Quarter (i.e., the Manufacturing Cost-Based Component of Supply Price paid or payable to uniQure + Net Sales Compensation), (c) the amount of withholding taxes, if any, required by Applicable Law to be deducted with respect to such Total Compensation, (d) the amount of Net Sales Compensation, if any, payable hereunder, (e) the number of units and average selling price (ASP) by product stock keeping unit (SKU) for the Products included in Net Sales for such Calendar Quarter, and (f) any other information reasonably requested by uniQure to assess the calculation of the Total Compensation and Net Sales Compensation.

**8.8 Payment Method.** All payments due under this Agreement shall be made by electronic funds transfer in immediately available funds to an account designated by the receiving Party. All payments hereunder shall be made in Dollars.

**8.9 Taxes.** Subject to Section 8.8, uniQure will pay any and all taxes levied on account of all payments it receives under this Agreement. If laws or regulations require that taxes be withheld with respect to any payments by BMS to uniQure under this Agreement, BMS will: (a) deduct those taxes from the remittable payment, (b) pay the taxes to the proper taxing authority, and (c) send evidence of the obligation together with proof of tax payment to uniQure on a timely basis following that tax payment. Each Party agrees to cooperate with the other Party in claiming refunds or exemptions from such deductions or withholdings under any relevant agreement or treaty which is in effect. The Parties shall discuss applicable mechanisms for minimizing such taxes to the extent possible in compliance with Applicable Law. uniQure will provide to BMS certain specified uniQure financial information reasonably requested by BMS that is required by BMS under Applicable Law for tax reporting purposes. In addition, the Parties shall cooperate in accordance with Applicable Law to minimize indirect taxes (such as value added tax, sales tax, consumption tax and other similar taxes) in connection with this Agreement. Any value added tax, sales tax, consumption tax and other similar taxes applicable to any payments hereunder shall be paid by the paying Party.

**8.10 Total Compensation on Sublicensee Sales.** BMS shall have the responsibility to account for and report sales of any Product by its Affiliates and Sublicensees on the same basis as if such sales were Net Sales by BMS. BMS shall pay to uniQure such amounts attributable to sales of its Affiliates and Sublicensees when due under this Agreement.

**8.11 Foreign Exchange.** Conversion of sales recorded in local currencies to Dollars shall be performed in a manner consistent with BMS' normal practices used to prepare its audited financial statements for internal and external reporting purposes.

**8.12 Offsetting.** BMS may offset any damages awarded to BMS under the Supply Agreement against any amounts payable to uniQure under this Agreement.

**8.13 Records.** BMS shall keep, and shall cause its Affiliates and Sublicensees to keep, complete, true and accurate books of accounts and records sufficient to determine and establish the amounts payable incurred under this Agreement, and compliance with the other terms and conditions of this Agreement. Such books and records shall be kept reasonably accessible and shall be made available for inspection for a three (3) year period in accordance with Section 8.14 below.

**8.14 Inspection of BMS Records.**

(a) **Inspection Payments.** Upon reasonable prior notice, BMS shall permit an

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independent nationally recognized certified public accounting firm (subject to obligations of confidentiality to BMS), appointed by uniQure and reasonably acceptable to BMS, to inspect the applicable records of BMS to the extent relating to payments to uniQure (including to verify the Total Compensation reports and the Total Compensation payments); *provided however*, that such inspection shall not occur more often than once per Calendar Year (for the current and preceding Calendar Year), unless a material error is discovered as part of such inspection in which case uniQure shall have the right to conduct a more thorough inspection for such period. Any inspection conducted under this Section 8.14(a) shall be at the expense of uniQure, unless such inspection reveals any underpayment of the Total Compensation due hereunder for the audited period by at least [\*\*], in which case the full costs of such inspection for such period shall be borne by BMS. Any underpayment shall be paid by BMS to uniQure within [\*\*] days with interest on the underpayment at the rate specified in Section 8.15 from the date such payment was originally due, and any overpayment shall be credited against future amounts due by BMS to uniQure.

(b) **Dispute Resolution.** In the event of a dispute regarding the findings of the inspection of the applicable records of BMS pursuant to Section 8.14(a) (including the Total Compensation reports and amount of Total Compensation payments owed to uniQure hereunder), the Parties shall work in good faith to resolve the dispute in accordance with the provisions of Section 16.1. If the Parties are unable to resolve the dispute within the time period stipulated in Section 16.1, the dispute shall be submitted for decision to a certified public accounting firm mutually selected by each Party's certified public accountants or to such other Third Party as the Parties shall mutually agree. The decision of such expert shall be final and the costs of such decision as well as the initial audit shall be borne between the Parties in such manner as such Expert shall determine. Not later than [\*\*] days after such decision and in accordance with such decision, BMS shall make any additional payments to uniQure, or uniQure shall reimburse any excess payments to BMS, as applicable. For clarity, any dispute subject to dispute resolution pursuant to this Section 8.14(b) shall not be subject to the terms and conditions of Sections 16.2 to 16.5 and any dispute regarding BMS' compliance with its obligation to use Diligent Efforts in the Development and Commercialization of Therapeutics and Products shall be subject to the terms and conditions of Article 16.

**8.15 Late Payments.** Any payments or portions thereof due hereunder that are not paid on the date such payments are due under this Agreement shall bear interest at a monthly rate equal to the lesser of: (a) [\*\*] percentage point above the monthly prime rate as published by Citibank, N.A., New York, New York, U.S. or any successor thereto, at 12:01 a.m. on the first day of each Calendar Quarter in which such payments are overdue or (b) the maximum monthly rate permitted by Applicable Law; in each case calculated on the number of days such payment is delinquent, compounded monthly.

**8.16 Payments to or Reports by Affiliates.** Any payment required under any provision of this Agreement to be made to either Party or any report required to be made by any Party shall be made to or by an Affiliate of that Party if designated in writing by that Party as the appropriate recipient or reporting entity.

**8.17 Inventor Compensation.** As between the Parties, each Party shall be responsible for and shall bear all costs associated with any Inventor Compensation, if any, for any employees of such Party or any of its Affiliates (or of any Third Party contractors of such Party or any of its Affiliates), whether employed at any time prior to the Effective Date or during the Term of this Agreement. For the avoidance of doubt, for purposes of this Section 8.17, uniQure shall not be considered a Third Party contractor of BMS, and BMS shall not be considered a Third Party contractor of uniQure. In addition, as between the Parties, uniQure shall be responsible for and shall bear all costs associated with any Inventor Compensation for any other developers or inventors of the

## 9. PATENT PROSECUTION AND ENFORCEMENT

### 9.1 Ownership of Information and Inventions.

(a) **Sole Inventions.** Each Party will own all right, title and interest in and to any inventions (and Patents that claim such inventions) solely invented by or on behalf of such Party, its Affiliates, or their respective employees, agents and independent contractors in the course of conducting any activities under this Agreement (collectively, “**Sole Inventions**”) and any Information generated or conceived solely by such Party, its Affiliates, or their respective employees, agents and independent contractors in the course of conducting any activities under this Agreement.

(b) **Joint Inventions.** Both Parties will jointly own all right, title and interest in and to any inventions invented jointly by the Parties, their Affiliates, or their respective employees, agents and independent contractors in the course of conducting any activities under this Agreement (collectively, “**Joint Inventions**”), Joint Patents and any Information generated or conceived jointly by the Parties, their Affiliates, or their respective employees, agents and independent contractors in the course of conducting any activities under this Agreement.

(c) **Use and Exploitation.** Subject to a Party’s obligations under applicable terms of this Agreement (e.g., licenses granted hereunder, confidentiality obligations, etc.) or Applicable Law with respect to same, any Sole Invention, Joint Invention, Joint Patent and Information (except to the extent containing Information first provided by the other Party) generated or resulting from a Party’s activities under this Agreement may be used or exploited by such Party for any purpose. This Agreement will be understood to be a joint research agreement under 35 U.S.C. §102(c) entered into for the purpose of researching, identifying and developing Therapeutics and Products under the terms set forth herein. Subject to and without limiting the licenses granted under this Agreement and the other terms and conditions of this Agreement and Applicable Law, neither Party shall otherwise have an obligation to account to the other, or obtain the consent of the other, with respect to the use or exploitation (directly or through licensees or other Third Parties) of any Sole Inventions of such Party, Joint Inventions, Joint Patents or Information of such Party.

### 9.2 Prosecution of Product Specific Patents.

#### (a) Prosecution by BMS.

(i) Subject to Section 9.2 (a)(ii) below, BMS will have the first right, but not the obligation, to draft, file, prosecute and maintain (including any oppositions, interferences, reissue proceedings, reexaminations and post-grant proceedings) in all jurisdictions in the Territory the Product Specific Patents (such activities with respect to Patents being the “**Prosecution**”, with the term “**Prosecute**” having the corresponding meaning; the Party that is responsible for Prosecuting a particular Patent is referred to as the “**Prosecuting Party**”). Such Prosecution of the Product Specific Patents shall be handled by outside counsel mutually agreed upon by the Parties that will jointly represent the Parties (the “**Patent Firm**”). Subject to Section 9.2(b) and (c), BMS shall [\*\*], and shall have [\*\*]. For clarity, each Party will [\*\*].

(ii) If BMS, either directly or through the Patent Firm, wishes to file a Patent which contains one or more claims that Cover the composition, formulation, method of use and method of manufacture of either (aa) one or more particular Therapeutics or Products (where such claim specifically discloses the Collaboration Target of such Therapeutic or Product) or (bb) one or more Therapeutics or Products as well as one or more Target Therapeutics or products that are not Therapeutics or Products (where such claim not only specifically discloses the Collaboration Target of such Therapeutic or Product but also any other Target), then BMS shall inform uniQure about such envisaged Patent filing in such manner and timing as to reasonably allow for uniQure to file one or more Patents for such claims or subject matters that

are directed solely to the method and/or process of manufacture. With regard to such claims or subject matters, uniQure shall be entitled to draft and file own Patents and, if uniQure wishes to file such own Patent, the Parties shall file their respective Patents on the same date. [\*\*].

(b) **Prosecution by uniQure.** In the event that BMS elects not to Prosecute or to cease Prosecution in any jurisdiction in the Territory any Product Specific Patent, BMS will give uniQure at least thirty (30) days written notice (*provided however*, that BMS shall provide at least sixty (60) days written notice where possible) before expiration of any relevant deadline and provide to uniQure information it reasonably requests relating to the Product Specific Patent. uniQure will then have the right, but not the obligation, to assume responsibility, using the Patent Firm, for the Prosecution of such Product Specific Patent in such jurisdiction. If uniQure assumes responsibility for the Prosecution of any such Product Specific Patents as set forth above, then (i) [\*\*] and (ii) if it relates to a Collaboration Target, Therapeutics or Products that are then the subject of a license to BMS under Section 7.1(a), then [\*\*].

(c) **Cooperation.** The Parties will cooperate in such Prosecution of the Product Specific Patents in all respects. Each Party will provide the other Party all reasonable assistance and cooperation in such Prosecution efforts, including providing any necessary powers of attorney and executing any other required documents or instruments for such Prosecution; *provided however*, that the Prosecuting Party shall reimburse the other Party for any out-of-pocket costs incurred by the other Party in providing such assistance and cooperation, where such costs and activities associated with such costs have been agreed to in advance by the Prosecuting Party. Each Party will provide the other Party with copies of any documents it receives or prepares in connection with such Prosecution and will inform the other Party of the progress of it. Before filing in connection with such Prosecution any document with a patent office, each Party will provide a copy of the document to the other Party sufficiently in advance to enable the other Party to review and comment on it, and the first Party will give due consideration to such comments. In particular, each Party agrees to provide the other Party with all information necessary or desirable to enable the other Party to comply with the duty of candor/duty of disclosure requirements of any patent authority.

(d) **Patent Term Adjustments or Extensions.** The Parties will confer regarding the desirability of seeking in any jurisdiction in the Territory any patent term adjustment, patent term extension, supplemental patent protection or related extension of rights with respect to the Product Specific Patents. Neither Party will proceed with such an adjustment, extension or protection until the Parties have consulted with one another and agreed to a strategy



therefor; *provided however*, that in the case where the Parties are unable to reach consensus, BMS will have the final decision-making authority with respect to such decision in jurisdictions where BMS Prosecutes any Product Specific Patents pursuant to Section 9.2(a), and uniQure will have the final decision-making authority with respect to such decision in jurisdictions where uniQure Prosecutes any Product Specific Patents pursuant to Section 9.2(b); *provided further*, that such decision will be made in accordance with Applicable Law so as to maximize marketing exclusivity for the Products in the Field. Each Party will cooperate fully with and provide all reasonable assistance to the other Party and use all reasonable efforts consistent with its obligations under Applicable Law (including any applicable consent order or decree) in connection with obtaining any such adjustment, extension or protection for the Product Specific Patents consistent with such strategy. To the extent reasonably and legally required in order to obtain any such adjustment, extension or protection in a particular jurisdiction, each Party will make available to the other a copy of the necessary documentation to enable such other Party to use the same for the purpose of obtaining the adjustment, extension or protection in such jurisdiction.

**9.3 Data Exclusivity.** As applicable, BMS will have the sole right and authority for securing, maintaining and enforcing exclusivity rights that may be available under Applicable Law in a country for a Product, such as any data, market, pediatric, orphan drug or other regulatory exclusivity periods. uniQure will cooperate fully with and provide all reasonable assistance to BMS and use all reasonable efforts consistent with its obligations under Applicable Law (including any applicable consent order or decree) to seek, maintain and enforce all data exclusivity periods available for the Products.

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#### **9.4 Prosecution of Other Patents.**

(a) **Joint Patents That Are Not Product Specific Patents and Not Other uniQure Patents.** This Section 9.4(a) will apply only to Joint Patents that are not included in (i) the Product Specific Patents and (ii) the Other uniQure Patents (the “**Other Joint Patents**”).

(i) **Prosecution by BMS.** BMS will have the first right, but not the obligation, to Prosecute in all jurisdictions in the Territory all such Other Joint Patents. Such Prosecution of the Other Joint Patents shall be handled by the Patent Firm. Subject to Section 9.4(a)(ii) and Section 9.4(a)(iii), BMS shall bear [\*\*] of the Patent Prosecution Costs for the Other Joint Patents, and shall have lead responsibility and decision-making control working with the Patent Firm for such Prosecution of the Other Joint Patents. For clarity, each Party will bear its own internal costs (i.e., those costs that are not Patent Prosecution Costs) with respect to its Prosecution activities for the Other Joint Patents.

(ii) **Prosecution by uniQure.** In the event that BMS elects not to Prosecute or to cease Prosecution in any jurisdiction in the Territory any Other Joint Patent, BMS will give uniQure at least thirty (30) days written notice (*provided however*, that BMS shall provide at least sixty (60) days written notice where possible) before expiration of any relevant deadline and provide to uniQure information it reasonably requests relating to the Other Joint Patent. uniQure will then have the right, but not the obligation, to assume responsibility, using the Patent Firm, for the Prosecution of such Other Joint Patent in such jurisdiction. If uniQure assumes responsibility for the Prosecution of any such Other Joint Patents as set forth above, then the Patent Prosecution Costs incurred by BMS in the course of such Prosecution will thereafter be borne by uniQure.

(iii) **Cooperation; Patent Term Adjustments or Extensions.** Section 9.2(c) and (d) shall apply *mutatis mutandis*.

(b) **BMS Patents.** BMS will have the sole right and authority with respect to BMS Patents in any jurisdiction, including Prosecution and Enforcement. BMS will be responsible for all costs incurred by it (including all Patent Prosecution Costs) in the course of Prosecuting and Enforcing such BMS Patents.

(c) **Other uniQure Patents.** As between the Parties, uniQure will have the first right, but not the obligation, to Prosecute in all jurisdictions all uniQure Patents other than the Product Specific Patents (including Joint Patents that are considered uniQure Patents pursuant to the terms of this Agreement and Patents that constitute uniQure Platform Technology or uniQure Platform Technology Improvements) (the “**Other uniQure Patents**”).

(i) **Prosecution by uniQure.** uniQure will have the first right, but not the obligation, to Prosecute in all jurisdictions all such Other uniQure Patents. Such Prosecution of the Other uniQure Patents shall be handled by the Patent Firm. Subject to Section 9.4(c)(ii) and Section 9.4(c)(iii), uniQure shall bear [\*\*] of the Patent Prosecution Costs for the Other uniQure Patents, and shall have lead responsibility and decision-making control working with the Patent Firm for such Prosecution of the Other uniQure Patents. For clarity, each Party will bear its own internal costs (i.e., those costs that are not Patent Prosecution Costs) with respect to its Prosecution activities for the Other uniQure Patents.

(ii) **Cooperation; Patent Term Adjustments or Extensions.** Section 9.2(c) and (d) shall apply *mutatis mutandis*.

#### **9.5 Infringement by Third Parties.**

(a) **Notification.** The Parties will promptly notify each other of any actual, threatened, alleged or suspected infringement by a Third Party of any of the Product Specific Patents, Other Joint Patents

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or Other uniQure Patents (an “**Infringement**”). A notice under 42 U.S.C. §262(l) (however such section may be amended from time to time during the Term) or a cause of action under 35 U.S.C. §271(e)(2)(c) (however such section may be amended from time to time during the Term), and any foreign equivalent thereof, with respect to a Product will be deemed to describe an act of Infringement, regardless of its content. As permitted by Applicable Law, each Party will promptly notify the other Party in writing of any such Infringement of which it becomes aware, and will provide evidence in such Party’s possession demonstrating such Infringement. In particular, each Party will notify and provide the other Party with copies of any allegations of patent invalidity, unenforceability or non-infringement of any Product Specific Patent or Other uniQure Patent Covering a Therapeutic or Product (including methods of use or manufacture thereof). Such notification and copies will be provided by the Party receiving such certification to the other Party as soon as practicable and, unless prohibited by Applicable Law, at least within five (5) Business Days after the receiving Party receives such certification. Such notification and copies will be sent by facsimile and overnight courier to BMS at the address set forth below, and to uniQure at the address specified in Section 17.5.

(b) **Infringement of Product Specific Patents.** BMS will have the first right, but not the obligation, to bring and control, at its expense, an appropriate suit or other action before any government or private tribunal against any Person allegedly engaged in any Infringement (an “**Infringement Action**”) of any Product Specific Patent to remedy the Infringement or to settle or otherwise secure the abatement of such Infringement (such activities with respect to Patents being the “**Enforcement**”, with the term “**Enforce**” having the corresponding meaning; the Party that is responsible for Enforcing a particular Patent is referred to as the “**Enforcing Party**”). The foregoing right of BMS shall include the right to perform all actions of a reference product sponsor set forth in 42 USC 262(l). uniQure will have the right, at its own expense and by counsel of its choice, to be represented in any Infringement Action with respect to a Product Specific Patent (“**Product Specific Infringement Action**”). BMS will have a period of ninety (90) days after its receipt or delivery of notice and evidence pursuant to Section 9.5(a) to elect to so Enforce such Product Specific Patents in the applicable jurisdiction (or to settle or otherwise secure the abatement of such Infringement); *provided however*, that such period will be more than ninety (90) days to the extent Applicable Law prevents earlier enforcement of such Product Specific Patents (such as the enforcement process set forth in 42 USC 262(l)) and such period will be less than ninety (90) days to the extent that a delay in bringing an action to enforce the applicable Product Specific Patents against such alleged Third Party infringer would limit or compromise the remedies (including monetary and injunctive relief) available against such alleged Third Party infringer. In the event BMS does not so elect to Enforce (or to settle or otherwise secure the abatement of such Infringement) within the aforementioned period of time, it will so notify uniQure in writing and in the case where uniQure then desires to commence a suit or take action to Enforce (or to settle or otherwise secure the abatement of such Infringement) the applicable Product Specific Patents with respect to such Infringement in the applicable jurisdiction, subject to Section 9.14, the Parties will confer and uniQure will have the right to commence such a suit or take such action to Enforce (or to settle or otherwise secure the abatement of such Infringement) the applicable Product Specific Patents, at uniQure’s expense. Each Party will provide to the Party Enforcing any such rights under this Section 9.5(b) reasonable assistance in such Enforcement, at such Enforcing Party’s request and expense, including joining such action as a party (and, at BMS’ request and expense, uniQure will use reasonable efforts to cause any applicable Existing Third Party Licensor to join such action as a party) if doing so is necessary for the purposes of establishing standing or is otherwise required by Applicable Law to pursue such action. The Enforcing Party will keep the other Party

regularly informed of the status and progress of such Enforcement efforts, and will reasonably consider the other Party’s comments on any such efforts.

(c) **Settlement.** Without the prior written consent of the other Party (not to be unreasonably withheld, conditioned or delayed), neither Party will settle any Product Specific Infringement Action in any manner that would adversely affect a Product Specific Patent or that would limit or restrict the ability of BMS (or its Affiliates or Sublicensees, as applicable) to manufacture or Commercialize Products anywhere in the Territory.

(d) **Expenses and Recoveries.** A Party bringing a Product Specific Infringement Action under this Section 9.5 against any Third Party engaged in Infringement of any Product Specific Patents will be [\*\*]. If such Party recovers monetary damages from such Third Party in such Product Specific Infringement Action, such recovery will [\*\*]. If such recovery is insufficient to cover all such costs and expenses of both Parties, it will [\*\*]. If after such reimbursement any funds remain from such damages, such funds will be shared as follows: (i) [\*\*], and (ii) [\*\*].

## 9.6 Infringement of Other Joint Patents.

(a) **Enforcement.** BMS will have the first right, but not the obligation, to bring and control, at its expense, an Infringement Action of any Other Joint Patents to remedy the Infringement or to settle or otherwise secure the abatement of such Infringement. uniQure will have the right, at its own expense and by counsel of its choice, to be represented in any Infringement Action with respect to an Other Joint Patent. BMS will have a period of ninety (90) days after its receipt or delivery of notice and evidence pursuant to Section 9.5(a) to elect to so Enforce such Other Joint Patent in the applicable jurisdiction (or to settle or otherwise secure the abatement of such Infringement); *provided however*, that such period will be more than ninety (90) days to the extent Applicable Law prevents earlier enforcement of such Other Joint Patents (such as the enforcement process set forth in 42 USC 262(l)) and such period will be less than ninety (90) days to the extent that a delay in bringing an action to enforce the applicable Other Joint Patents against such alleged Third Party infringer would limit or compromise the remedies (including monetary and injunctive relief) available against such alleged Third Party infringer. In the event BMS does not so elect to Enforce (or to settle or otherwise secure the abatement of such Infringement) within the aforementioned period of time, it will so notify uniQure in writing and in the case where uniQure then desires to commence a suit or take action to Enforce (or to settle or otherwise secure the abatement of such Infringement) the applicable Other Joint Patents with respect to such Infringement in the applicable jurisdiction, the Parties will confer and uniQure will have the right to commence such a suit or take such action to Enforce (or to settle or otherwise secure the abatement of such Infringement) the applicable Other Joint Patents, at uniQure’s expense. Each Party will provide to the Party Enforcing any such rights under this Section 9.6(a) reasonable assistance in such Enforcement, at such Enforcing Party’s request and expense, including joining such action as a party if doing so is necessary for the purposes of establishing standing or is otherwise required by Applicable Law to pursue such action. The Enforcing Party will keep the other Party regularly informed of the status and progress of such Enforcement efforts, and will reasonably consider the other Party’s comments on any such efforts.

(b) **Settlement.** Without the prior written consent of the other Party (not to be unreasonably withheld, conditioned or delayed), neither Party will settle any Infringement Action of any Other Joint Patents in any manner that would adversely affect an Other Joint Patent.

(c) **Expenses and Recoveries.** A Party bringing an Infringement Action of any Other Joint Patents under Section 9.6(a) against any Third Party engaged in Infringement of any Other Joint Patent will be [\*\*]. If such Party recovers monetary damages from such Third Party in such Infringement Action, such recovery will [\*\*]. If such recovery is [\*\*]. If after such reimbursement [\*\*], such funds will be shared as follows: (i) [\*\*], and (ii) [\*\*].

## 9.7 Infringement of Other uniQure Patents.

(a) **Enforcement.** uniQure will have the first right, but not the obligation, to bring and control, at its expense, an Infringement Action of any Other uniQure Patents to remedy the Infringement or to settle or otherwise secure the abatement of such Infringement. BMS will have the right, at its own expense and by counsel of its choice, to be represented in any Infringement Action with respect to an Other uniQure Patent. uniQure will have a period of ninety (90) days after its receipt or delivery of notice and evidence pursuant to Section 9.5(a) to elect to so Enforce such Other uniQure Patent in the applicable jurisdiction (or to settle or otherwise secure the abatement of such Infringement). In the event uniQure does not so elect to Enforce (or to settle or otherwise secure the abatement of such Infringement) within the aforementioned period of time, it will so notify BMS in writing and in the case where BMS then desires to commence a suit or take action to Enforce (or to settle or otherwise secure the abatement of such Infringement) the applicable Other uniQure Patents with respect to such Infringement in the applicable jurisdiction, subject to Section 9.14, the Parties will confer and BMS will have the right to commence such a suit or take such action to Enforce (or to settle or otherwise secure the abatement of such Infringement) the applicable Other uniQure Patents, at BMS' expense. Each Party will provide to the Party Enforcing any such rights under this Section 9.7(a) reasonable assistance in such Enforcement, at such Enforcing Party's request and expense, including joining such action as a party if doing so is necessary for the purposes of establishing standing or is otherwise required by Applicable Law to pursue such action. The Enforcing Party will keep the other Party regularly informed of the status and progress of such Enforcement efforts, and will reasonably consider the other Party's comments on any such efforts.

(b) **Settlement.** Without the prior written consent of the other Party (not to be unreasonably withheld, conditioned or delayed), neither Party will settle any Infringement Action of any such Other uniQure Patent in any manner that would adversely affect an Other uniQure Patent or limit or restrict the ability of BMS (or its Affiliates or Sublicensees, as applicable) to manufacture or Commercialize Products anywhere in the Territory.

(c) **Expenses and Recoveries.** A Party bringing an Infringement Action of any Other uniQure Patents under Section 9.7(a) against any Third Party engaged in Infringement of any Other uniQure Patent will be [\*\*]. If such Party recovers monetary damages from such Third Party in such Infringement Action, such recovery will [\*\*]. If such recovery is insufficient to cover all such costs and expenses of both Parties, it will be [\*\*]. If after such reimbursement any funds remain from such damages, such funds will be shared as follows: (i) [\*\*] and (ii) [\*\*].

## 9.8 Third Party Rights.

(a) The Parties will promptly notify each other of any written allegation that any activity pursuant to this Agreement infringes the Patent rights of any Third Party.

(b) Subject to Section 9.8(c), (d) and (e), with respect to any Third Party Patent rights under Section 9.8(a), Section 7.8 shall apply.

(c) Notwithstanding the foregoing, in the case a claim of infringement of a Patent is brought against a Party in a suit or other action or proceeding with respect to any Third Party Patent rights under Section 9.8(a), such Party will have the right, at its own expense and by counsel of its own choice, to prosecute and defend any such claim in such suit or other action or proceeding. If both Parties are named, the Parties shall meet and determine who is best situated to lead any such suit or other action or proceeding.

(d) Without the prior written consent of the other Party (not to be unreasonably withheld, conditioned or delayed), neither Party will settle any claim under this Section 9.8 in any manner that would (i) limit or restrict the ability of BMS (or its Affiliates or Sublicensees, as applicable) to manufacture or Commercialize Products anywhere in the Territory or (ii) impose any obligation, restriction

or limitation on the other Party.

(e) The Parties will cooperate in all respects with one another in prosecuting or defending any action pursuant to this Section 9.8.

## 9.9 Reexaminations, Oppositions, Post-Grant Trial Proceedings and Related Actions.

(a) The Parties will promptly notify each other in the event that any Third Party files, or threatens to file, any paper in a court, patent office or other Governmental Authority, seeking to invalidate, review the patentability, reexamine, oppose or compel the licensing of any Product Specific Patent, any Other Joint Patent or any Other uniQure Patent contemplated to be used or practiced under in the conduct of the Research Program (any such Third Party action being a "**Patent Challenge**").

(b) BMS will have the first right to bring and control, at its expense, any effort in defense of such a Patent Challenge against a Product Specific Patent or Other Joint Patent, except in the case where such Patent Challenge is made in connection with an Infringement Action then currently being Enforced, in which case the Enforcing Party in the Infringement Action will have the first right to bring and control the defense of such Patent Challenge and such Patent Challenge will be considered part of the Infringement Action under this Article 9. In the case where BMS controls the defense of such Patent Challenge, uniQure will have the right, at its own expense and by counsel of its choice, to be represented in any such effort. If BMS fails to take action to defend such Patent Challenge within thirty (30) days of the time limit for bringing such defense (or within such shorter period to the extent that a delay in bringing such defense would limit or compromise the outcome of such defense of such Patent Challenge), then, subject to Section 9.14, uniQure will have the right, but not the obligation, to bring and control any effort in defense of such Patent Challenge at its own expense.

(c) uniQure will have the first right to bring and control, at its expense, any effort in defense of such a Patent Challenge against an Other uniQure Patent, except in the case where such Patent Challenge is made in connection with an Infringement Action then currently being Enforced, in which case the Enforcing Party in the Infringement Action will have the first right to bring and control the defense of such Patent Challenge and such Patent Challenge will be considered part of such Infringement Action under this Article 9. In the case where uniQure controls the defense of such Patent Challenge, BMS will have the right, at its own expense and by counsel of its choice, to be represented in any such effort. If uniQure fails to take action to defend such Patent Challenge within thirty (30) days of the time limit for bringing such defense (or within such shorter period to the extent that a delay in bringing such defense would limit or compromise the outcome of such defense of such Patent Challenge), then, subject to Section 9.14, BMS will have the right, but not the obligation, to bring and control any effort in defense of such Patent Challenge at its own expense.

**9.10 Disclosure of Inventions.** To the extent relating to any Collaboration Target, Therapeutic or Product (including methods of making a Therapeutic), each Party will promptly disclose to the other Party all invention disclosures submitted to such Party by its or its Affiliates' employees describing Joint Inventions and Sole Inventions. Each Party will also respond promptly to reasonable requests from the other Party for more Information relating to such inventions.

**9.11 Patent Contacts.** Each Party will designate patent counsel representatives who will be responsible for coordinating the activities between the Parties in accordance with this Article 9 (each a “**Patent Contact**”). Each Party will designate its initial Patent Contact within thirty (30) days following the Effective Date and will promptly thereafter notify the other Party of such designation. If at any time, a vacancy occurs for any reason, the Party that appointed the prior incumbent will as soon as reasonably practicable appoint a successor. Each Party will promptly notify the other Party of any substitution of another person as its Patent Contact. The Patent Contacts will, from time to time, coordinate the respective patent strategies of the Parties relating to this Agreement. In particular the Patent Contacts will review and

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update the list of uniQure Patents from time to time to ensure that all Products being Developed or Commercialized are covered.

**9.12 Personnel Obligations.** Prior to receiving any Confidential Information or beginning work under this Agreement relating to any discovery or research of a Collaboration Target or to any discovery, research, Development or Commercialization of a Therapeutic or a Product, each employee, agent or independent contractor of BMS or uniQure or of either Party’s respective Affiliates will be bound in writing by invention assignment obligations which are consistent with the obligations of BMS or uniQure under this Agreement; *provided however*, that where necessary in the case of a Third Party, such Third Party shall agree to grant BMS or uniQure, as the case may be, an exclusive license with the right to grant sublicenses with respect to resulting inventions and Patents. Notwithstanding the preceding sentence, to the extent the Applicable Law in a country prohibits binding in writing any employee, agent or independent contractor of a Party (or its Affiliates) in such country by invention assignment obligations, such Party (or its Affiliates) shall not be required to comply with the requirement in the preceding sentence to obtain written invention assignment obligations, such Party (or its Affiliates) shall, to the extent applicable, claim any invention arising under this Agreement from any employee, agent or independent contractor of such Party in accordance with the Applicable Law in such country, such Party (or its Affiliates) shall not release any invention arising under this Agreement to any employee, agent or independent contractor of such Party without the prior written consent of the other Party, and such Party (or its Affiliates) shall be solely responsible for any Inventors Compensation due to any such employee, agent or independent contractor for any invention arising under this Agreement.

**9.13 Further Action.** Each Party shall, upon the reasonable request of the other Party, provide such assistance and execute such documents as are reasonably necessary for such other Party to exercise its rights or perform its obligations pursuant to this Article 9; *provided however*, that neither Party shall be required to take any action pursuant to this Article 9 that such Party reasonably determines in its sole judgment and discretion conflicts with or violates any applicable order of a Governmental Authority or Applicable Law.

**9.14 Disputes Regarding Valid Claim and Inventorship.** As set forth in the definition of Valid Claim, disputes between the Parties as to whether a claim of a pending application within the uniQure Patents is a Valid Claim shall be resolved by arbitration pursuant to Section 16.2 with the/each arbitrator being a patent attorney having appropriate expertise in patent law. In addition, any dispute between the Parties regarding the inventorship of Sole Inventions and Joint Inventions shall be resolved by arbitration pursuant to Section 16.2 with the/each arbitrator being a patent attorney having appropriate expertise in patent law. Accordingly, such disputes under this Section 9.14 shall be treated as a Dispute subject to resolution under Section 16.2. In the case of any such dispute under this Section 9.14, prior to any such arbitration, the Parties shall first meet (through their Patent Contacts and other representatives) and discuss in good faith a possible resolution of such dispute.

## 10. TRADEMARKS

**10.1 Product Trademarks.** BMS shall be solely responsible for the selection (including the creation, searching and clearing), registration, maintenance, policing and enforcement of all trademarks developed for use in connection with the Commercialization of Products in the Field in the Territory (the “**Product Marks**”). BMS or its Affiliates shall own all Product Marks, and all trademark registrations for said marks.

**10.2 Use of Name.** Neither Party shall, without the other Party’s prior written consent, use any trademarks or other marks of the other Party or such other Party’s Affiliates (including the other Party’s corporate name), or any trademarks or other marks, advertising taglines or slogans confusingly similar thereto, in connection with such Party’s Commercialization of Products under this Agreement or for any

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other purpose, except to the extent required in connection with activities under this Agreement or to comply with Applicable Law.

**10.3 Further Actions.** Each Party shall, upon the reasonable request of the other Party, provide such assistance and execute such documents as are reasonably necessary for such other Party to exercise its rights or perform its obligations pursuant to this Article 10; *provided however*, that neither Party shall be required to take any action pursuant to this Article 10 that such Party reasonably determines in its sole judgment and discretion conflicts with or violates any applicable order of a Governmental Authority or Applicable Law.

## 11. EXCLUSIVITY

**11.1 Exclusivity.** uniQure agrees that it will not work independently of this Agreement on its own behalf, or through, with or on behalf of any Affiliate or Third Party, or grant any license, option or other right to any Affiliate or Third Party, or enable any Affiliate or Third Party, or transfer any uniQure Know-How, in each case

(a) during the Research Term (i.e., the Initial Research Term, as may be extended by the Parties pursuant to Section 3.1(e)), [\*\*];

(b) during the Research Term (i.e., the Initial Research Term, as may be extended by the Parties pursuant to Section 3.1(e)) and subject to Sections 3.3(b)(iii), 3.3(d)(iii), 3.3(d)(iv) and 3.3(e), with respect to any Reserved Target or any Variant thereof, [\*\*] for any [\*\*] and any [\*\*];

(c) for as long as a [\*\*], with respect to discovery, research, Development or Commercialization activities in the Field in the Territory with respect to such [\*\*], any [\*\*]; and

(d) for as long as [\*\*].

The foregoing obligations shall [\*\*], and with respect to [\*\*], subparagraphs (a) and (d) shall apply but not subparagraphs (b) and (c).

If uniQure or any of its Affiliates merges or consolidates with, is otherwise acquired by, or acquires, a Third Party (including through a Change of Control Transaction), the foregoing obligations shall not apply with respect to any activities, projects and/or programs of such an acquirer or acquired Third Party or other Third Party in which uniQure is consolidated or merged, *provided, however*, that in any such case (i) [\*\*], (ii) the exclusivity obligations under this Section 11 [\*\*], (iii) uniQure (if uniQure is not a surviving entity) shall [\*\*], (iv) if uniQure is a surviving entity, uniQure shall [\*\*], and (v) if uniQure is not a surviving entity, uniQure shall [\*\*].

## 12. CONFIDENTIALITY

**12.1 Confidentiality.** Except to the extent expressly authorized by this Agreement or otherwise agreed in writing by the Parties, each Party (the “**Receiving Party**”) agrees that, for the Term and for [\*\*] years thereafter, it shall keep confidential and shall not publish or otherwise disclose, and shall not use for any purpose other than as provided for in this Agreement (which includes the exercise of any rights or the performance of any obligations hereunder), any Confidential Information furnished to it by or on behalf of the other Party (the “**Disclosing Party**”) or such other Party’s Affiliates pursuant to this Agreement, except for that portion of such Confidential Information that the Receiving Party can demonstrate by competent written proof:

(a) was already known to the Receiving Party or any of its Affiliates, other than under an obligation of confidentiality to the Disclosing Party or any of its Affiliates, at the time of its disclosure to

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the Receiving Party or any of its Affiliates;

(b) was generally available to the public or otherwise part of the public domain at the time of its disclosure to the Receiving Party or any of its Affiliates;

(c) becomes generally available to the public or otherwise part of the public domain after its disclosure to the Receiving Party or any of its Affiliates and other than through any act or omission of the Receiving Party or any of its Affiliates in breach of this Agreement;

(d) is subsequently disclosed to the Receiving Party or any of its Affiliates by a Third Party without obligations of confidentiality to the Disclosing Party or any of its Affiliates with respect thereto; or

(e) is subsequently independently discovered or developed by the Receiving Party or any of its Affiliates without the aid, application, or use of Confidential Information of the Disclosing Party or any of its Affiliates.

To the extent relating specifically to Therapeutics or Products, all Information generated by uniQure or its Affiliates in the performance of the Research Program shall be treated as the Confidential Information of BMS for so long as this Agreement is in effect with respect to such Therapeutics and Products. Each Party agrees that with respect to its employees, agents, independent contractors and any Sublicensees, that it shall provide or permit access to Confidential Information of the other Party to its employees, agents, independent contractors and any Sublicensees who have a need to know such Confidential Information to assist the receiving Party with the activities contemplated or required of it by this Agreement and who are subject to obligations of confidentiality and non-use with respect to such Confidential Information substantially similar to the obligations of confidentiality and non-use of the receiving Party pursuant to this Section 12.1; *provided* that each Party shall remain responsible for any failure by any such recipient to treat such Confidential Information as required under this Section 12.1.

**12.2 Authorized Disclosure.** Each Party may disclose Confidential Information belonging to the other Party to the extent such disclosure is reasonably necessary in the following situations:

(a) filing or prosecuting Patents in accordance with this Agreement, including pursuant to Article 9;

(b) subject to Section 12.3, regulatory filings and other filings with Governmental Authorities (including Regulatory Authorities), including filings with the FDA and EMA, as necessary for the Development or Commercialization of a Therapeutic or Product, or as required in connection with any filing, application or request for Regulatory Approval; *provided however*, that reasonable measures will be taken to assure confidential treatment of such information;

(c) prosecuting or defending arbitration or litigation;

(d) subject to Section 12.3, complying with Applicable Law, including regulations promulgated by the SEC or other relevant securities exchanges;

(e) disclosure to its Affiliates, or its or its Affiliates’ employees, agents, independent contractors and any Sublicensees only on a need-to-know basis and solely in connection with the performance of this Agreement;

(f) disclosure of the stage of Development of Therapeutics or Products under this Agreement to any bona fide potential or actual investor, stockholder, investment banker, acquirer, merger

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partner or other potential or actual financial partner only on a need-to-know basis and solely in connection with this Agreement;

(g) disclosure of the material terms of this Agreement to any bona fide potential or actual investor, stockholder, investment banker, acquirer, merger partner or other potential or actual financial partner only on a need-to-know basis and solely in connection with this Agreement;

(h) disclosure pursuant to Section 12.5;

(i) in the case of uniQure, disclosure to any Existing Third Party Licensors in connection with any Existing License Agreements only on a need-to-know basis in connection with this Agreement;

(j) disclosure to its legal or financial advisors only on a need-to-know basis and solely in connection with this Agreement,

*provided however*, in case of disclosure pursuant to Sections 12.2(e), 12.2(f), 12.2(g) or 12.2(i) each disclosee must be bound by obligations of confidentiality and non-use at least as equivalent in scope as and no less restrictive than those set forth in this Article 12 prior to any such disclosure; *provided further*, that the term of such disclosee's obligations regarding the use and disclosure of Confidential Information shall be as long as can be reasonably negotiated with such disclosee, but in any event no less than [\*\*] years after the date of disclosure to the disclosee; *provided further*, that the disclosing Party shall remain responsible for any failure by any such disclosee to treat such Confidential Information as required under this Article 12.

Notwithstanding the foregoing, in the event a Party is required to make a disclosure of the other Party's Confidential Information pursuant to Sections 12.2(a), 12.2(c) or 12.2(d), it will, except where impracticable, give reasonable advance written notice to the other Party of such disclosure and use reasonable efforts to secure confidential treatment of such Confidential Information. In any event, the Parties agree to take all reasonable action to avoid disclosure of Confidential Information hereunder.

Nothing in Sections 12.1 or 12.2 shall limit either Party in any way from disclosing to any Third Party that is a tax authority, the Party's outside tax or legal advisors, or the Party's independent auditors, such Party's U.S. or foreign income tax treatment and the U.S. or foreign income tax structure of the transactions relating to such Party that are based on or derived from this Agreement, as well as all materials of any kind (including opinions or other tax analyses) relating to such tax treatment or tax structure, except to the extent that nondisclosure of such matters is reasonably necessary in order to comply with applicable securities laws.

### 12.3 Publicity; Terms of Agreement.

(a) The Parties agree that the material terms of this Agreement are the Confidential Information of both Parties, subject to the special authorized disclosure provisions set forth in Section 12.2 and this Section 12.3. Except as set forth in Sections 12.3(b) and 12.3(c), each Party agrees not to issue any press release or other public announcement disclosing the terms of this Agreement or the transaction contemplated hereby without the prior written consent of the other Party. The Parties have agreed that uniQure may make a public announcement of the execution of this Agreement substantially in the form of the press release attached as **Exhibit I** on or after the Effective Date. uniQure agrees to provide BMS at least twenty-four (24) hours' notice prior to issuing such press release.

(b) Each Party may disclose the terms of this Agreement in the case of a press release or governmental filing (including any prospectus in connection with an IPO or other securities offering) to the extent required by Applicable Law, including regulations promulgated by the SEC or other relevant

securities exchanges (where reasonably advised by the disclosing Party's counsel); *provided however*, that the disclosing Party shall, except where impracticable, give reasonable advance written notice of the proposed text of such release or filing to the other Party for its prior review but shall not be required to obtain approval therefor.

(c) The Parties acknowledge that either or both Parties may be obligated to file under Applicable Law a copy of this Agreement with the SEC or other Governmental Authorities. Each Party shall be entitled to make such a required filing; *provided however*, that it requests confidential treatment of at least the financial terms and sensitive technical terms hereof and thereof to the extent such confidential treatment is reasonably available to such Party. In the event of any such filing, each Party will provide the other Party with a copy of this Agreement marked to show provisions for which such Party intends to seek confidential treatment not less than five (5) Business Days prior to such filing (and any revisions to such portions of the proposed filing a reasonable time prior to the filing thereof), and shall reasonably consider the other Party's comments thereon to the extent consistent with the legal requirements, with respect to the filing Party, governing disclosure of material agreements and material information that must be publicly filed, and shall only disclose Confidential Information which it is advised by counsel or the applicable Governmental Authority is legally required to be disclosed. No such notice shall be required under this Section 12.3(c) if the substance of the description of or reference to this Agreement contained in the proposed filing has been included in any previous filing made by either Party hereunder or otherwise approved by the other Party.

(d) Each Party shall require each of its Affiliates and private investors to which Confidential Information of the other Party is disclosed as permitted hereunder to comply with the covenants and restrictions set forth in Section 12.1 through Section 12.3 as if each such Affiliate and each such investor were a Party to this Agreement and shall be fully responsible for any breach of such covenants and restrictions by any such Affiliate or investor.

**12.4 Publications.** Neither Party shall publicly present or publish results of studies carried out under this Agreement (each such presentation or publication a "**Publication**") without the opportunity for prior review by the other Party, except to the extent otherwise required by Applicable Law, in which case Section 12.3 shall apply with respect to disclosures required by the SEC or other Governmental Authorities or stock exchanges or for regulatory filings. The submitting Party shall provide the other Party the opportunity to review any proposed Publication at least thirty (30) days prior to the earlier of its presentation or intended submission for publication. The submitting Party agrees, upon request by the other Party, not to submit or present any Publication until the other Party has had thirty (30) days to comment on any material in such Publication. The submitting Party shall consider the comments of the other Party in good faith, but will retain the sole authority to submit the manuscript for Publication; *provided however*, that the submitting Party agrees to delay such Publication as necessary to enable the Parties to file a Patent if a Party reasonably believes that such Publication might adversely affect such Patent. The submitting Party shall provide the other Party a copy of the Publication at the time of the submission or presentation. Notwithstanding the foregoing, BMS shall not have the right to publish or present uniQure's Confidential Information without uniQure's prior written consent, and uniQure shall not have the right to publish or present BMS' Confidential Information without BMS' prior written consent. Each Party agrees to acknowledge the contributions of the other Party, and the employees of the other Party, in all publications as scientifically appropriate. This Section 12.4 shall not limit and shall be subject to Section 12.5.

Nothing contained in this Section 12.4 shall prohibit the inclusion of information in a Patent application claiming, and in furtherance of, the manufacture, use, sale or formulation of a Collaboration Target, Therapeutic or Product; *provided however*, that the non-filing Party is given a reasonable opportunity to review, comment upon or approve the information to be included prior to submission of such Patent application, where and to the extent required by Article 9 hereof. Notwithstanding the foregoing, the Parties recognize that independent investigators have been engaged, and will be engaged in the future, to conduct Clinical Trials of Therapeutics and Products. The Parties recognize that such investigators operate in an

academic environment and may release information regarding such studies in a manner consistent with academic standards; *provided however*, that each Party will use reasonable efforts to prevent publication prior to the filing of relevant Patent applications and to ensure that no Confidential Information of either Party is disclosed; *provided further*, that without limiting the foregoing, Prof. [\*\*] and Prof. [\*\*] shall have the publication rights set forth in **Exhibit M**.

**12.5 Publication and Listing of Clinical Trials and Compliance with other Policies, Orders and Agreements.** Each Party agrees to comply, with respect to the Therapeutics and Products and to the extent applicable to its activities conducted under this Agreement, with (a) the Pharmaceutical Research and Manufacturers of America (PhRMA) Guidelines on the listing of Clinical Trials and the Publication of Clinical Trial results, (b) any applicable court order, stipulations, consent agreements and settlements entered into by such Party, and (c) BMS' Research and Development policy concerning Clinical Trials Registration and Disclosure of Results as amended from time to time and other BMS policies or other policies adopted by it for the majority of its other pharmaceutical products with regard to the same (to the extent the same either are not in direct conflict with the documents referred to in clauses (a) and (c) above and, in the case of uniQure, to the extent all such relevant policies in clause (c) are provided by BMS to uniQure in writing prior to requiring their implementation under this Agreement).

**12.6 Effect of Change of Control.** If uniQure or any of its Affiliates merges or consolidates with, is otherwise acquired by, or acquires, a Third Party (including through a Change of Control Transaction), [\*\*].

**12.7 Prior CDA.** As of the Effective Date all Confidential Information (as that term is defined in the Prior CDA) exchanged between the Parties and their Affiliates under the Prior CDA with respect to uniQure's heart failure program referred to in Schedule A of the Prior CDA shall be deemed Information exchanged under this Agreement, shall be subject to the terms and conditions of this Agreement, and shall no longer be subject to the terms and conditions of the Prior CDA. uniQure and BMS agree that, as of the Effective Date, the Prior CDA is terminated and superseded by this Agreement with respect to such Confidential Information with respect to uniQure's heart failure program referred to in Schedule A of the Prior CDA. The Prior CDA shall remain in effect and shall apply to all other Confidential Information (as that term is defined in the Prior CDA) exchanged between the Parties under the Prior CDA, including Confidential Information with respect to uniQure's hemophilia program that is referenced in Schedule A of the Prior CDA.

### 13. TERM AND TERMINATION

**13.1 Term.** This Agreement shall become effective on the Effective Date and, unless earlier terminated pursuant to this Article 13, shall continue, on a Product-by-Product and country-by-country basis until such time as neither Party has any obligation to the other under this Agreement in such country with respect to such Product (the "**Term**").

#### 13.2 Termination by BMS at Will or for Safety Reasons.

(a) **Termination by BMS at Will.** BMS may terminate this Agreement [\*\*] prior written notice to uniQure in the case where, at the time BMS serves such termination notice, Regulatory Approval has not been obtained for any applicable Product with respect to such Collaboration Target in either the U.S. or the EU, or upon [\*\*]; *provided however*, such notice may be provided no earlier than the third anniversary of the Effective Date.

(b) **Termination by BMS for Safety Reasons.** BMS may terminate this Agreement [\*\*] upon written notice to uniQure based on Safety Reasons. Termination under this Section 13.2(b) for a particular Collaboration Target shall include any and all Therapeutics and Products for such Collaboration Target. Termination under this Section 13.2(b) for a particular Therapeutic shall include any and all

Products related to such Therapeutic. Upon such termination for Safety Reasons, BMS (or the applicable Affiliate or Sublicensee of BMS) shall be responsible, at its expense, for the wind-down of any Development of the applicable Therapeutic or Product (including any Clinical Trials for the applicable Product being conducted by or on behalf of BMS) and any Commercialization activities for the applicable Product. Such termination shall become effective upon the date that BMS notifies uniQure in writing that such wind-down is complete, but in any event no later than six (6) months after uniQure has received the termination from BMS pursuant to sentence 1 of this Section 13.2(b). Following any such notice of termination under this Section 13.2(b), [\*\*]. Instead, if uniQure notifies BMS in writing that it wants to pursue the research, Development and/or Commercialization of any Therapeutic, Product or Collaboration Target terminated for Safety Reasons, BMS will only be required to provide the data generated for such Therapeutic, Product or Collaboration Target, BMS will withdraw or have withdrawn all Regulatory Materials for such Therapeutic or Product, uniQure will have the right to re-file the data provided by BMS, and no royalty or any other payment will be due to BMS for the research, Development or Commercialization of such Therapeutic, Product or Collaboration Target by uniQure.

#### 13.3 Termination for Breach.

(a) **Termination Notice.** Subject to Sections 13.3(b) to (d) and Section 13.4, either Party may terminate this Agreement with respect to any Product (on a Product-by-Product basis) as to the entire Territory or, at the discretion of the terminating Party, with respect to any country (on a country-by-country basis), in the event the other Party materially breaches this Agreement, and such breach shall have continued for sixty (60) days (or, in the event that the default is a non-payment default that cannot be cured within such sixty (60) day period, if the alleged breaching Party has not commenced and diligently continued good faith efforts to cure such breach within such sixty (60) day period) after written notice shall have been provided to the breaching Party by the non-breaching Party requiring such breach to be remedied (a "**Termination Notice**"). Except as set forth in Section 13.3(b), any such termination shall become effective at the end of such sixty (60) day period unless the breaching Party has cured any such breach prior to the expiration of the sixty (60) day period (or, if such default cannot be cured within such sixty (60) day period, if the alleged breaching Party has not commenced to cure such breach prior to the expiration of the sixty (60) day period and diligently continued good faith efforts to cure such breach after the expiration of the sixty (60)

day period). Any such Termination Notice shall state the terminating Party's intent to terminate and the reasons and justification for such termination and, at the sole discretion of the terminating Party, may include recommended steps which the terminating Party believes the breaching Party should take to cure such alleged breach.

(b) **Dispute as to Breach.** If the alleged breaching Party disputes the existence or materiality of a breach specified in a Termination Notice provided by the other Party in accordance with Section 13.3(a), and such alleged breaching Party provides the other Party notice of such dispute within said sixty (60) day period after receiving such Termination Notice, then the termination of the non-breaching Party shall not become effective under Section 13.3(a) with respect to the applicable Product and country or countries unless and until the existence of such material breach by BMS has been determined in accordance with Article 16 and BMS fails to cure such breach within sixty (60) days following such determination (except to the extent such breach involves the failure to make a payment when due, which breach must be cured within ten (10) Business Days following decision of such arbitrator(s)). It is understood and agreed that during the pendency of such dispute, all of the terms and conditions of this Agreement shall remain in effect and the Parties shall continue to perform all of their respective obligations hereunder.

(c) **Not Applicable to Diligence Breach.** This Section 13.3 shall not apply to or encompass a breach (or alleged breach) of BMS' obligation to use Diligent Efforts as set forth in Sections 3.9, 4.5 or 5.1, and for which a right to terminate, if any, for any such breach shall be governed solely by Section 13.4.

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(d) **Milestones Owed.** Where uniQure is the alleged breaching Party, [\*\*], during the period between the notice of termination under Section 13.3(a) and the effective date of termination; *provided however*, that [\*\*].

(e) **Termination for BMS Patent Challenge.** Except to the extent the following under this Section 13.3(e) is unenforceable under the Applicable Law of the applicable jurisdiction where the applicable uniQure Patent is issued, [\*\*] (a "**BMS Patent Challenge**") through any administrative, judicial or other similar proceeding with respect to an issued uniQure Patent in a particular jurisdiction, [\*\*]. For the avoidance of doubt, uniQure shall [\*\*], and this Section 13.3(e) shall not apply, with respect to (i) [\*\*] or (ii) [\*\*]. In the case [\*\*].

### 13.4 Termination by uniQure for Failure of BMS to Use Diligent Efforts.

(a) **Notice of and Right to Terminate.** Subject to Sections 13.6 and 13.7, uniQure shall have the right to terminate this Agreement:

(i) on a [\*\*] basis with respect to a Collaboration Target if BMS is in material breach either of (x) its obligation to use Diligent Efforts as set forth in Section 4.5 with respect to the Development of a Therapeutic or Product with respect to such Collaboration Target, or (y) its obligations to use Diligent Efforts as set forth in Section 5.1 with respect to the Commercialization of a Product with respect to such Collaboration Target, [\*\*]; *provided however*, that this Agreement shall not so terminate unless (A) BMS is given written notice by uniQure (which notice shall state uniQure's intent to terminate and the reasons and justification for such termination and, at the sole discretion of uniQure, may include recommended steps which uniQure believes BMS should take to cure such alleged breach), and (B) BMS, or its Affiliates or Sublicensee, has not (1) during the sixty (60) day period following receipt of such notice by BMS, provided uniQure with a plan for the diligent Development or Commercialization of a Therapeutic or, as the case may be, Product with respect to such Collaboration Target in the Field in such [\*\*] consistent with its obligations under Sections 4.5 and 5.1 and (2) during the six (6) month period following such notice carried out such plan and cured such alleged breach by diligently pursuing the Development or Commercialization of a Therapeutic or, as the case may be, Product with respect to such Collaboration Target in the Field in such [\*\*] as set forth in Sections 4.5 and 5.1.

(ii) on a [\*\*] basis if BMS is in material breach of its obligations under Section 3.9 with respect to its obligations under the Research Program as they relate to such [\*\*]; *provided however*, that this Agreement shall not so terminate unless (A) BMS is given written notice by uniQure (which notice shall state uniQure's intent to terminate and the reasons and justification for such termination and, at the sole discretion of uniQure, may include recommended steps which uniQure believes BMS should take to cure such alleged breach), and (B) BMS, or its Affiliates, has not (1) during the sixty (60) day period following receipt of such notice by BMS, provided uniQure with a plan for the cure of such alleged breach consistent with its obligations under Section 3.9 and (2) during the six (6) month period following such notice carried out such plan and cured such alleged breach (or, if such default cannot be cured within such six (6) months period, if BMS has not commenced to cure such breach prior to the expiration of the six (6) months period and diligently continued good faith efforts to cure such breach after the expiration of the six (6) months period).

(b) **Dispute as to Failure.** If BMS disputes in good faith the allegation that it has failed to use requisite Diligent Efforts or is otherwise in material breach or failure of its obligations as specified in a notice provided by uniQure pursuant to Section 13.4(a), and if BMS provides notice to uniQure of such dispute within the sixty (60) days following receipt of such notice provided by uniQure, then the termination of uniQure shall not become effective under Section 13.4(a) with respect to the applicable [\*\*], respectively, unless and until the existence of such material breach or failure by BMS has been determined in accordance with Article 16 and BMS fails to cure such breach within sixty (60) days following such determination. Except as set forth in Section 13.4(c), it is understood and agreed that during the pendency of such dispute,

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all of the terms and conditions of this Agreement shall remain in effect and the Parties shall continue to perform all of their respective obligations hereunder.

(c) **Interpretation.** For clarity and subject to Section 13.6, any termination pursuant to Section 13.4(a)(i) shall be [\*\*]), and accordingly any such termination shall have no effect on the remainder of this Agreement and shall not in any way limit the rights and obligations of the Parties under this Agreement with respect to [\*\*]. For clarity, any termination pursuant to Section 13.4(a)(ii) shall be for [\*\*]), and accordingly any such termination shall have no effect on the remainder of this Agreement and shall not in any way limit the rights and obligations of the Parties under this Agreement with respect to any other Collaboration Target.

### 13.5 Other Termination Rights.



(a) **Termination for Insolvency.** A Party shall have the right to terminate this Agreement upon written notice if the other Party incurs an Insolvency Event; *provided however*, that in the case of any involuntary bankruptcy proceeding, such right to terminate shall only become effective if the Party that incurs the Insolvency Event consents to the involuntary bankruptcy or if such proceeding is not dismissed or stayed within forty-five (45) days after the filing thereof. “**Insolvency Event**” means circumstances under which a Party (a) has a receiver or similar officer appointed over all or a material part of its assets or business; (b) passes a resolution for winding-up of all or a material part of its assets or business (other than a winding-up for the purpose of, or in connection with, any solvent amalgamation or reconstruction) or a court enters an order to that effect; (c) has entered against it an order for relief recognizing it as a debtor under any insolvency or bankruptcy laws (or any equivalent order in any jurisdiction); or (d) enters into any composition or arrangement with its creditors with respect to all or a material part of its assets or business (other than relating to a solvent restructuring).

(b) **Termination for Failure to Receive Regulatory Approval from Competition Authorities.** In the event that regulatory approval is not received from the U.S. Federal Trade Commission, the U.S. Department of Justice, or any relevant foreign Governmental Authority in response to any required competition filings with respect to the transactions contemplated on or before November 1, 2015, either Party may terminate this Agreement forthwith by written notice to the other Party, unless such failure shall be due to (a) the failure of the Party requesting termination to provide all of the information required pursuant to the HSR Act or any applicable foreign equivalent thereof in the initial filing or upon additional requests from the appropriate regulators; or (b) the failure of the Party requesting termination to fulfill any of the conditions set forth in Section 17.16(b) and 17.16(c) by the Termination Date.

### 13.6 Limitations on Termination Remedy.

(a) **Right to Terminate in ROW.** Notwithstanding anything herein to the contrary, in the event that uniQure terminates, or has the right to terminate, this Agreement pursuant to Section 13.4(a)(i) with respect to a [\*\*], then uniQure shall [\*\*].

(b) **Termination in EU Countries.** Notwithstanding anything herein to the contrary, (i) in the event that uniQure has the right to terminate this Agreement pursuant to Section 13.4(a)(i) with respect to a Collaboration Target with respect to [\*\*].

(c) **No Effect on Other Collaboration Targets.** For the avoidance of doubt, any termination under this Article 13 with respect to a particular Collaboration Target shall have no effect on and shall not in any way limit the licenses granted under this Agreement to BMS for Therapeutics and Products with respect to any other Collaboration Target.

**13.7 Effects of Termination of this Agreement.** Upon termination of this Agreement by BMS under Section 13.2(a) or by uniQure under Section 13.3, Section 13.4 or Section 13.5 (except as the

application of such Sections may be limited as provided in a given subsection of this Section 13.7), the following shall apply with respect to the terminated Collaboration Targets in each of the terminated country(ies) (in addition to any other rights and obligations under this Agreement with respect to such termination, *provided, however*, that the rights and remedies set forth in this Section 13.7 (if the terminating Party is uniQure) or in Section 13.8 (if the terminating Party is BMS) shall be the sole and exclusive remedies of the terminating Party for breach of this Agreement by the other Party).

(a) **Terminated Therapeutics and Targets.** Upon termination of this Agreement with respect to a Collaboration Target with respect to a country, such terminated Collaboration Target (the “**Terminated Target**”) shall no longer be considered to be a Collaboration Target with respect to such country, and all rights licensed by uniQure to BMS under Section 7.1 with respect to such country shall terminate and revert to uniQure pursuant to Section 13.7(c). “**Terminated Therapeutics**” means the Therapeutics with respect to such Terminated Target, *provided however*, that Terminated Therapeutics shall exclude in any case (i) [\*\*]; (iii) [\*\*]; (iv) [\*\*]; and (v) [\*\*]. “**BMS Proprietary Target**” means a Target that has not been [\*\*]. “**Terminated Products**” means Products containing such Terminated Therapeutics.

(b) **Provision of uniQure Know-How and Information.** Upon such Collaboration Target becoming a Terminated Target, no later than sixty (60) days after the effective date of termination, (i) BMS shall return to uniQure all uniQure Know-How and other Information provided by uniQure in tangible form with respect to the applicable Terminated Targets, Terminated Therapeutics and Terminated Products, and (ii) BMS shall further provide uniQure all reports and other Information in the possession and Control of BMS and generated with respect to such Terminated Targets, Terminated Therapeutics or Terminated Products under the Research Program (except for Regulatory Materials, which are covered by Section 13.7(e)).

(c) **Termination of License from uniQure.** The rights and licenses granted to BMS in this Agreement, including under Section 7.1, shall terminate solely with respect to each terminated Collaboration Target as well as any Therapeutic and/or Product for such terminated Collaboration Target (independent whether such terminated Collaboration Target is considered a Terminated Target or a BMS Proprietary Target pursuant to Section 13.7(a)) in the country(ies) in which the termination becomes effective; *provided however*, that BMS shall retain a non-exclusive license under Section 7.1 in the terminated country(ies) to sell and offer for sale the Terminated Products during the Commercialization Wind-Down Period in accordance with Section 13.7(d) (including the right to sell such Terminated Products through BMS Sublicensees if BMS were using such Sublicensees to sell same prior to such termination date). For clarity, Terminated Targets, Terminated Therapeutics and Terminated Products shall no longer be subject to the exclusivity restrictions set forth in Article 11.

(d) **Commercialization Wind-Down.** BMS, its Affiliates and Sublicensees shall be entitled to continue to sell (but not to actively promote after the effective date of termination) any existing inventory of Terminated Products in each terminated country of the Territory for which Regulatory Approval therefor has been obtained (provided that such Products shall have launched in each such terminated country as of the applicable effective date of termination), in accordance with the terms and conditions of this Agreement for a period of [\*\*] (the “**Commercialization Wind-Down Period**”), and any Terminated Products sold or disposed of during this Commercialization Wind-Down Period shall be subject to the same Total Compensation as would have applied had this Agreement otherwise remained in full force and effect with respect to such Terminated Products. Following such Commercialization Wind-Down Period, BMS, its Affiliates and Sublicensees shall not sell such Terminated Products in such terminated country(ies) or make any representation regarding BMS’ status as a licensee of such Terminated Products in such country(ies).

(e) **Regulatory Materials.** Upon uniQure’s written request, BMS shall assign to uniQure or uniQure’s designee(s) all right, title and interest in, and provide uniQure or uniQure’s designee(s) with original copies of, any Regulatory Materials (including Regulatory Approvals) for the Terminated

Therapeutics and Terminated Products in the terminated country(ies) that are held or Controlled by or under authority of BMS, its Affiliates or Sublicensees, that are necessary or reasonably useful for the continued Development or Commercialization of the Terminated Targets or Terminated Products in the terminated country(ies). BMS shall either assign to uniQure or uniQure's designee(s), or provide uniQure or uniQure's designee(s) with a right of reference with respect to such Regulatory Materials, as uniQure determines at its reasonable discretion. In addition, upon uniQure's written request, BMS shall provide to uniQure copies of all material related documentation, including material preclinical and clinical data that are held or Controlled by or under authority of BMS, its Affiliates or Sublicensees with respect to the Terminated Therapeutics or Terminated Products. The Parties shall discuss and establish appropriate arrangements with respect to safety data exchange.

(f) **Return of Confidential Information.** Within thirty (30) days after the effective date of a termination or, if a Commercialization Wind-Down Period is applicable, after the end of the Commercialization Wind-Down Period, BMS shall destroy all tangible items comprising, bearing or containing any Confidential Information of uniQure that are in BMS' or its Affiliates' possession or control and that relate to each terminated Collaboration Target as well as any Therapeutic and/or Product for such terminated Collaboration Target (independent whether such terminated Collaboration Target is considered a Terminated Target or a BMS Proprietary Target pursuant to Section 13.7(a)) in the country(ies) in which the termination becomes effective, and provide written certification of such destruction, or prepare such tangible items of Confidential Information for shipment to uniQure, as uniQure may direct, at BMS' expense; *provided however*, that BMS may retain one (1) copy of such Confidential Information for its legal archives; and *provided further* that BMS shall not be required to destroy electronic files containing Confidential Information that are made in the ordinary course of its business information back-up procedures pursuant to its electronic record retention and destruction practices that apply to its own general electronic files and information.

(g) **Transition Assistance.** BMS agrees to cooperate with uniQure and its designee(s), at uniQure's sole expense, to facilitate an orderly and prompt transition of the Development (and as applicable, Commercialization) activities with respect to the Terminated Targets, Terminated Therapeutics and Terminated Products in the terminated country(ies) to uniQure or its designee(s) following such termination. In addition, BMS agrees to (i) continue to manufacture and supply Terminated Therapeutics or Terminated Products, to the extent then being manufactured by BMS itself (and not through a CMO) for the terminated country(ies), to uniQure or its designee(s), at a supply price to be paid by uniQure to BMS equal to BMS' fully absorbed cost, for a period of up to [\*\*] following the effective date of termination or (ii) where such Terminated Therapeutics or Terminated Products are then being manufactured on BMS' behalf by a CMO, shall use reasonable efforts to cause such CMO to continue to supply and deliver to uniQure or its designee(s), at the same cost as was provided to BMS by such CMO, such Terminated Therapeutics or Terminated Products for a period of [\*\*], and to undertake reasonable efforts to assign to uniQure the relevant CMO agreements, in whole or in part, under which the Terminated Therapeutics or Terminated Products are then being manufactured for the terminated country(ies), or facilitate uniQure's entry into similar agreements, in each case, until such time as uniQure or its designee(s) have completed the transition of the Development (and as applicable, Commercialization) activities with respect to the Terminated Therapeutics and Terminated Products in the terminated country(ies), but in no case longer than [\*\*] following the effective date of termination. In the event of such supply of Terminated Therapeutics or Terminated Products by or for BMS, the Parties shall enter into a supply agreement having terms regarding such supply essentially equivalent to those of the Supply Agreement. For the avoidance of doubt, uniQure shall be responsible for the conduct at its expense of all Development (and as applicable, Commercialization) activities for each Terminated Therapeutic and each Terminated Product as of the applicable termination date under Section 13.7(a).

(h) **Royalty to BMS.** Unless this Agreement is terminated by uniQure pursuant to Sections 13.3, 13.4 or 17.8(c) or by BMS under Section 13.2(b) (in which case no royalty shall be owed by

uniQure), uniQure shall pay BMS a royalty equal to [\*\*] of net sales of such Product in the applicable terminated Major Markets by uniQure or uniQure's Affiliates, licensees or sublicensees, *provided however*, that such termination occurs any time after [\*\*]. For purposes of this Section, "net sales" shall be calculated in the same manner Net Sales are defined for sales made by BMS, substituting "uniQure, its Affiliates and (sub)licensees" for each reference to a Related Party in such Section, and the provisions of Article 8 of this Agreement shall apply to uniQure (as royalty payor) and BMS (as royalty recipient) with respect to such royalties in the same manner as such provisions had applied to a Related Party (as Net Sales Compensation payor) and uniQure (as Net Sales Compensation recipient).

(i) **Rights and Licenses to uniQure.** The licenses granted to uniQure pursuant to Section 7.3 and the other rights and licenses granted to uniQure pursuant to this Agreement shall survive the termination until expiry of all Patent rights therein and shall extend under the same conditions also to any purpose inside the Field in the terminated country(ies). In particular, uniQure shall have the exclusive (even as to BMS) right and license, with the right to assign and grant sublicensees, to use any uniQure Know-How and other Information, Regulatory Materials (including Regulatory Approvals), documentation and data, Confidential Information of uniQure, and any other deliveries to be provided to uniQure in accordance with Section 13.7 to research, develop, make, use, sell, offer for sale, export and import (including the exclusive right to Develop and Commercialize) Terminated Targets, Terminated Therapeutics and Terminated Products in the Field in the terminated country(ies). BMS further grants to uniQure and its Affiliates an exclusive (even as to BMS), fully paid up, royalty-free, irrevocable, perpetual and unlimited license, with the right to assign and sublicense, under the relevant BMS Patents in the terminated country(ies) to research, develop, make, use, sell, offer for sale, export and import (including the exclusive right to Develop and Commercialize) Terminated Targets, Terminated Therapeutics and Terminated Products in the Field in the terminated country(ies).

(j) Where a terminated Collaboration Target is a BMS Proprietary Target and if uniQure wants to initiate any work on such Collaboration Target or any Therapeutics or Products for such Collaboration Target and such Collaboration Target was not terminated for any Safety Reasons, BMS will be willing to enter into discussions for a license under Information and Patents Controlled by BMS and its Affiliates that would be necessary to research, Develop and Commercialize Therapeutics and Products for such Collaboration Target.

**13.8 Effects of Termination of Agreement by BMS under Section 13.3(a) or Section 13.5.** Upon termination of this Agreement by BMS under Section 13.3(a) or Section 13.5 the following shall apply:

(a) all rights and licenses granted to BMS under this Agreement shall survive but shall become irrevocable and perpetual (subject to BMS' obligations under Article 8); and

(b) BMS shall have no further Diligent Efforts obligations under Sections 3.9, 4.5 or 5.1 with respect to the applicable Collaboration Target, Therapeutics and Products in the terminated country or countries.

**13.9 Effects of Expiration of Agreement.** Upon the expiration of the TC Term (i.e., in the case where there is no earlier termination pursuant to this Article 13), on a [\*\*], the licenses granted to BMS under Article 7 with respect to uniQure Technology shall be irrevocable, perpetual, royalty-free and fully paid-up.

**13.10 Other Remedies.** Termination or expiration of this Agreement for any reason shall not release either Party from any liability or obligation that already has accrued prior to such expiration or termination, nor affect the survival of any provision hereof to the extent it is expressly stated to survive such termination. In the event of a termination by BMS under Section 13.2(a) during the Research Term, BMS shall be responsible for the Research Program Costs that are included in the Budget for the period of three (3)

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months following the date such termination becomes effective to the extent that such Research Program Costs are actually incurred by uniQure, *provided however*, that uniQure shall use commercially reasonable efforts to cancel all relevant contracts and Third Party obligations and reallocate or return any unused materials, equipment and similar purchases so as to limit the Research Program Costs actually incurred by uniQure. Subject to and without limiting the terms and conditions of this Agreement (including Section 13.7 and Section 15.4), expiration or termination of this Agreement shall not preclude any Party from (a) claiming any other damages, compensation or relief that it may be entitled to upon such expiration or termination, (b) any right to receive any amounts accrued under this Agreement prior to the expiration or termination date but which are unpaid or become payable thereafter and (c) any right to obtain performance of any obligation provided for in this Agreement which shall survive expiration or termination.

**13.11 Survival.** Termination or expiration of this Agreement shall not affect rights or obligations of the Parties under this Agreement that have accrued prior to the date of termination or expiration of this Agreement. Notwithstanding anything to the contrary, the following provisions shall survive and apply after expiration or termination of this Agreement: Sections 3.3(d)(ii), 3.8, 3.10(b), 3.10(c), 3.11 (regarding non-disclosure and non-use obligations and responsibility for compliance), 3.14 (regarding non-disclosure and non-use obligations and responsibility for compliance), 3.15, 6.3 (regarding non-disclosure and non-use obligations), 7.3(b), 7.4, 7.5, 7.10, 8.4(b)(ii), 8.5(e) (the last sentence), 8.13-8.17, 9.1, 9.4(a) and (b), 9.5, 9.6, 9.14, 12.1, 12.2, 12.3 and Articles 1 (to the extent necessary to interpret other surviving sections), 13, 14, 15, 16 and 17. In addition, the other applicable provisions of Article 8 shall survive to the extent required to make final payments with respect to Net Sales incurred or accrued prior to the date of termination or expiration. All provisions not surviving in accordance with the foregoing shall terminate upon expiration or termination of this Agreement and be of no further force and effect.

**13.12 Breach of Agreement, Transaction Agreements or Supply Agreement.** Notwithstanding anything to the contrary in any of this Agreement, the Transaction Agreements or the Supply Agreement, and in addition to, and not in lieu of, any rights of a party of these agreements pursuant hereto or thereto, if the counterparty of any of these agreements materially breaches any provision in any of this Agreement, the Transaction Agreements or the Supply Agreement (pursuant to the terms of such agreement), the non-breaching party may by notice to the breaching party suspend the non-breaching party's performance under any or all provisions under this Agreement until such material breach is cured (pursuant to the terms of such agreement) and such suspension of performance shall not be deemed a breach of any obligation by the non-breaching party.

## 14. REPRESENTATIONS AND WARRANTIES

**14.1 Mutual Representations and Warranties and Covenants.** Each Party hereby represents and warrants as of the Signing Date and, where denoted below, covenants to the other Party as follows:

(a) It is a company or corporation duly organized, validly existing, and in good standing under the laws of the jurisdiction in which it is incorporated, and has full corporate power and authority and the legal right to own and operate its property and assets and to carry on its business as it is now being conducted and as contemplated in this Agreement.

(b) It has the full corporate power and authority and the legal right to enter into this Agreement and perform its obligations hereunder, including the right to grant the licenses granted by it hereunder. It has taken all necessary corporate action on its part required to authorize the execution and delivery of this Agreement and the performance of its obligations hereunder. This Agreement has been duly executed and delivered on behalf of such Party, and, subject to Section 17.16, constitutes a legal, valid, and binding obligation of such Party that is enforceable against it in accordance with its terms.

(c) It is not a party to any agreement, or, to the best of its knowledge, outstanding order, judgment or decree of any court or Governmental Authority that would prevent it from granting the rights

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granted to the other Party under this Agreement or performing its obligations under this Agreement.

(d) In the course of the Development of Therapeutics and Products, such Party has not used prior to the Signing Date, and shall not use during the Term, to the best of its knowledge, any employee, agent or independent contractor who has been debarred by any Governmental Authority, or, to the best of such Party's knowledge, is the subject of debarment proceedings by a Governmental Authority.

(e) It has not prior to the Signing Date, and will not during the Term, grant any right to any Third Party that would conflict with the rights granted to the other Party hereunder.

(f) The execution and delivery of this Agreement and the performance of such Party's obligations hereunder do not conflict with, violate, breach, or constitute a default, or require any consent not already obtained under any contractual obligation or, to the best of such Party's knowledge, court or administrative order by which such Party is bound.

(g) It (or its Affiliates) has and, during the Term will have, enforceable written agreements with all of its (or its Affiliates) employees who receive Confidential Information under this Agreement assigning to such Party (or its Affiliates) ownership of all intellectual property rights created in the course of their employment, unless such employees are obliged by Applicable Law to assign ownership of all such intellectual property rights to such

Party or ownership of all such intellectual property rights are automatically assigned by Applicable Law (including cases where such automatic assignment is subject to the relevant Party (or its Affiliates) claiming such intellectual property right in accordance with Applicable Law).

**14.2 Representations and Warranties and Covenants by uniQure.** uniQure hereby represents and warrants as of the Signing Date and, where denoted below, covenants to BMS as follows:

(a) Except as disclosed in **Exhibit R**, the uniQure Technology existing as of the Signing Date and licensed by uniQure to BMS under this Agreement is free and clear from any Liens. uniQure has sufficient legal or beneficial title, ownership or license under the uniQure Technology to grant the licenses to BMS as purported to be granted pursuant to this Agreement. As of the Signing Date, except for the Patents licensed to uniQure or its Affiliates under the Existing License Agreements, uniQure or its Affiliates are the sole owner of all right, title and interest in and to the uniQure Patents listed or identified in **Exhibit A** and **Exhibit B**. All fees required to maintain such issued uniQure Patents listed or identified in **Exhibit A** and **Exhibit B** have been paid as of the Signing Date.

(b) uniQure is not obligated to [\*\*], and hereby covenants not to (except as expressly permitted under this Agreement [\*\*]) [\*\*].

(c) Except as disclosed by uniQure in writing to BMS' Patent Contact prior to the Signing Date, uniQure has not received [\*\*]. To the best of uniQure's knowledge, the uniQure Technology existing as of the Signing Date was not [\*\*].

(d) Except as disclosed by uniQure in writing to BMS' Patent Contact prior to the Signing Date, to the best of uniQure's knowledge, the use of the uniQure Technology by uniQure to conduct the Research Program as contemplated as of the Signing Date to identify Collaboration Targets and the research, Development, Commercialization and manufacture of Target Therapeutics for Collaboration Targets, including the Lead S100A1 Therapeutic, [\*\*].

(e) Except as disclosed by uniQure in writing to BMS' Patent Contact prior to the Signing Date, [\*\*].

(f) To the best of uniQure's knowledge, there are no [\*\*].

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(g) To the best of uniQure's knowledge, it has a reasonable basis to conclude that [\*\*].

(h) uniQure has not granted, and uniQure covenants that during the Term it shall not grant (except in accordance with the express terms and conditions of this Agreement) any [\*\*]. uniQure has not granted, and uniQure covenants that during the Term it shall not grant (except in accordance with the express terms and conditions of this Agreement) to any Third Party [\*\*].

(i) To the extent not disclosed in **Exhibit A** or **Exhibit B**, uniQure has disclosed in writing to BMS' Patent Counsel prior to the Signing Date (i) all uniQure Patents existing as of the Signing Date and (ii) the jurisdiction(s) by or in which each such uniQure Patent has been issued or in which an application for such uniQure Patent has been filed, together with the respective patent or application numbers. All fees required to maintain such issued uniQure Patents disclosed to BMS' Patent Counsel have been paid as of the Signing Date.

(j) All inventors of any inventions included within the uniQure Technology that are existing as of the Effective Date (except for inventions to which uniQure or its Affiliates have obtained licenses or other rights from Third Parties, such as under the Existing License Agreements) have [\*\*]. To uniQure's knowledge, there are no claims that have been asserted in writing [\*\*].

(k) uniQure has maintained and, unless otherwise agreed to by BMS, will maintain and keep in full force and effect all agreements and filings (including Patent filings, in accordance with Article 9) necessary to perform its obligations hereunder. Neither uniQure nor its Affiliates are [\*\*] and, to the best of uniQure's knowledge, no other party to any Existing License Agreement [\*\*] in any respect thereunder.

(l) No Third Party [\*\*] under any agreement with uniQure that would reasonably be expected to interfere with BMS' exercise of its rights licensed under Section 7.1 hereof.

(m) Prior to the Signing Date, uniQure has not [\*\*], except (i) in connection with the preparation and filing of Patent applications for certain of the uniQure Patents or (ii) to a Third Party (e.g., patent counsel, regulatory and other advisors, pharmaceutical companies) subject to written or statutory confidentiality obligations with such Third Party.

(n) Prior to the Signing Date, [\*\*] (1) did not use in relation to any of its/their research and development activities concerning [\*\*] that was not in the public domain and available to be used by any Person free of charge and that was not licensed to [\*\*], with the right to sublicense (through multiple tiers), and (2) did not permit any Third Party to use any such Information, Patent or other intellectual property for itself or for, on behalf of or for the benefit of [\*\*].

(o) uniQure covenants that on and after the Signing Date, uniQure and its Affiliates (1) [\*\*], (2) [\*\*] (2) [\*\*].

**14.3 Obligations as of Effective Date.** Each Party shall use its reasonable efforts to ensure that its representations and warranties set forth in this Article 14 remain true and correct at and as of the Effective Date as if such representations and warranties were made at and as of the Effective Date.

**14.4 No Other Representations or Warranties.** EXCEPT AS EXPRESSLY STATED IN THIS ARTICLE 14, NO REPRESENTATIONS OR WARRANTIES WHATSOEVER, WHETHER EXPRESS OR IMPLIED, INCLUDING WARRANTIES OF MERCHANTABILITY, FITNESS FOR A PARTICULAR PURPOSE, NON-INFRINGEMENT OR NON-MISAPPROPRIATION OF THIRD PARTY INTELLECTUAL PROPERTY RIGHTS, OR THAT ANY OF THE DEVELOPMENT OR COMMERCIALIZATION EFFORTS WITH REGARD TO ANY COLLABORATION TARGET, THERAPEUTIC OR PRODUCT WILL BE SUCCESSFUL, IS MADE OR GIVEN BY OR ON BEHALF OF A PARTY. EXCEPT AS EXPRESSLY STATED IN THIS ARTICLE 14, ALL REPRESENTATIONS

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AND WARRANTIES, WHETHER ARISING BY OPERATION OF LAW OR OTHERWISE, ARE HEREBY EXPRESSLY EXCLUDED.

## 15. INDEMNIFICATION AND LIMITATION OF LIABILITY

**15.1 Indemnification by uniQure for Third Party Claims.** uniQure shall defend, indemnify, and hold BMS, its Affiliates, and their respective officers, directors, employees, and agents (the “**BMS Indemnitees**”) harmless from and against any and all damages or other amounts payable to a Third Party claimant, as well as any reasonable attorneys’ fees and costs of litigation incurred by such BMS Indemnitees, all to the extent resulting from any claims, suits, proceedings or causes of action brought by such Third Party (collectively, “**BMS Claims**”) against such BMS Indemnitees that arise out of or result from (or are alleged to arise out of or result from): (a) a breach of any of uniQure’s representations, warranties, covenants and obligations under this Agreement; (b) the gross negligence or willful misconduct of uniQure, its Affiliates, or the officers, directors, employees, or agents of uniQure or its Affiliates in the performance of this Agreement; (c) the research or Development of Target Therapeutics before the Effective Date; (d) the research, Development and/or Commercialization by uniQure or its Affiliates or (sub)licensees of any Terminated Target, Terminated Therapeutic or Terminated Product or of any Collaboration Target, Therapeutic or Product that was terminated by BMS under Section 13.2(b) or of any other Collaboration Target, Therapeutic or Product licensed to uniQure under this Agreement, (e) any breach by uniQure or its Affiliates of, or any failure by uniQure or its Affiliates, or their respective contractors or agents, to perform, observe or comply with any of the provisions of any Existing License Agreement or Third Party Agreement, (f) [\*\*] (g) the use by any Third Party of any such Information, Patent or other intellectual property for itself or for, on behalf of or for the benefit of Inocard GmbH, uniQure GmbH, uniQure and/or any Affiliate of uniQure where such Information, Patent or other intellectual property was provided to or purportedly licensed to such Third Party by uniQure and/or any Affiliate of uniQure, or (h) the use by BMS, any Affiliate of BMS and/or any Third Party as permitted under this Agreement of any such Information, Patent or other intellectual property provided to or purportedly licensed to BMS, any Affiliate of BMS and/or any Third Party by uniQure and/or any Affiliate of uniQure under this Agreement, except to the extent that such breach or failure is attributable to a breach by BMS or its Affiliates of any of BMS’ obligations under this Agreement or the Supply Agreement. The foregoing indemnity obligation shall not apply to the extent that any BMS Claim is subject to indemnity pursuant to Section 15.2 or is based on a breach by BMS or its Affiliates of an obligation under an agreement between BMS or its Affiliates and a Third Party.

**15.2 Indemnification by BMS for Third Party Claims.** BMS shall defend, indemnify, and hold uniQure, its Affiliates, and their respective officers, directors, employees, and agents (the “**uniQure Indemnitees**”) harmless from and against any and all damages or other amounts payable to a Third Party claimant, as well as any reasonable attorneys’ fees and costs of litigation incurred by such uniQure Indemnitees, all to the extent resulting from any claims, suits, proceedings or causes of action brought by such Third Party (collectively, “**uniQure Claims**”) against such uniQure Indemnitees that arise out of or result from (or are alleged to arise out of or result from): (a) the research, Development, manufacture, storage, handling, use, sale, offer for sale, exportation, importation or Commercialization of Collaboration Targets, Target Therapeutics, Therapeutics or Products under the Research Program by BMS, its Affiliates or Sublicensees (including product liability claims (including failures to warn, misuse and strict liability claims) and claims for infringement or misappropriation of Patents ([\*\*])), trademarks, trade secrets and other intellectual property rights, in each case to the extent such claim arises, directly or indirectly, out of or results from the research, Development, manufacture, storage, handling, use, sale, offer for sale, exportation, importation or Commercialization of Collaboration Targets, Target Therapeutics, Therapeutics or Products under the Research Program by BMS, its Affiliates or Sublicensees; (b) a breach of any of BMS’ representations, warranties, covenants and obligations under this Agreement; or (c) the gross negligence or willful misconduct of BMS, its Affiliates, or the officers, directors, employees, or agents of BMS or its Affiliates in the performance of this Agreement, or (d) any breach by BMS or its Affiliates of, or any failure by BMS or its Affiliates, or their respective contractors or agents, to perform, observe or comply with any of

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the provisions of any Existing License Agreement or Third Party Agreement applicable to BMS, except to the extent that such breach or failure is attributable to a breach by uniQure or its Affiliates of any of uniQure’s obligations under this Agreement or the Supply Agreement. The foregoing indemnity obligation shall not apply to the extent that any uniQure Claim is subject to indemnity pursuant to Section 15.1 or is based on a breach by uniQure or its Affiliates of an obligation under an agreement between uniQure or its Affiliates and a Third Party.

**15.3 Indemnification Procedures.** In order for a party claiming indemnity under this Article 15 (the “**Indemnified Party**”) to be entitled to any indemnification provided for under this Article 15, the Indemnified Party shall give written notice to the Party from whom indemnity is being sought (the “**Indemnifying Party**”) within ten (10) Business Days after learning of the claim, suit, proceeding or cause of action for which indemnity is being sought (“**Claim**”) (it being understood and agreed, however, that the failure or delay by an Indemnified Party to give such notice of a Claim shall not affect the indemnification provided hereunder except to the extent the Indemnifying Party shall have been prejudiced as a result of such failure or delay to give such notice). If the Indemnifying Party is not contesting the indemnity obligation, the Indemnified Party shall permit the Indemnifying Party to control and assume the defense of any litigation relating to such Claim and disposition of any such Claim, unless the Indemnifying Party is also a party (or likely to be named a party) to the proceeding in which such Claim is made and the Indemnified Party gives notice to the Indemnifying Party that it may have defenses to such Claim or proceeding that are in conflict with the interests of the Indemnifying Party, in which case the Indemnifying Party shall not be so entitled to assume the defense of the case. If the Indemnifying Party does assume the defense of any Claim, it (a) shall act diligently and in good faith with respect to all matters relating to the settlement or disposition of any Claim as the settlement or disposition relates to parties being indemnified under this Article 15; (b) shall cause such defense to be conducted by counsel reasonably acceptable to the Indemnified Party; (c) shall keep the Indemnified Party reasonably advised of the status of such Claim and the defense thereof and shall consider recommendations made by the Indemnified Party with respect thereto; and (d) shall not settle or otherwise resolve any Claim without prior notice to the Indemnified Party and the consent of the Indemnified Party if such settlement involves anything other than the payment of money by the Indemnifying Party. The Indemnified Party shall reasonably cooperate with the Indemnifying Party in its defense of any Claim for which the Indemnifying Party has assumed the defense in accordance with this Section 15.3, and shall have the right (at its own expense) to be present in person or through counsel at all legal proceedings giving rise to the right of indemnification. So long as the Indemnifying Party is diligently defending the Claim in good faith, the Indemnified Party shall not settle any such Claim without the prior written consent of the Indemnifying Party. If the Indemnifying Party does not assume and conduct the defense of the Claim as provided above, (y) the Indemnified Party may defend against, and consent to the entry of any judgment or enter into any settlement with respect to the Claim in any manner the Indemnified Party may deem reasonably appropriate (and the Indemnified Party need not consult with, or obtain any consent from, the Indemnifying Party in connection therewith), and (z) the Indemnifying Party will remain responsible to indemnify the Indemnified Party as provided in this Article 15.

**15.4 Shared Liability.** Any and all damages or other amounts payable to a Third Party claimant (other than those that would (a) if the Claim was or had been made against BMS, be covered by the indemnity provided for in Section 15.1 and (ii) if the Claim was or had been made against uniQure, be covered by the indemnity provided for in Section 15.2) in connection with any Claim for product liability brought against a Party or its Affiliates by such Third Party claimant after the Effective Date, to the extent resulting from the manufacture, use, handling, storage, sale or other disposition of any Product in

the Territory after the Effective Date (collectively, “**Product Liability Losses**”) shall be shared between the Parties in relation to their actual commercial benefit under this Agreement determined as of the point in time when such Claim is made.

**15.5 Limitation of Liability.** EXCEPT FOR (A) DAMAGES PAID OR PAYABLE TO A THIRD PARTY BY AN INDEMNIFIED PARTY FOR WHICH THE INDEMNIFIED PARTY IS

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ENTITLED TO INDEMNIFICATION UNDER THIS ARTICLE 15; (B) A BREACH BY UNIQUE OF ARTICLE 11; (C) A BREACH BY EITHER PARTY OF SECTION 12.1 OR 12.2; (D) DAMAGES THAT ARE ATTRIBUTABLE TO THE WILLFUL BREACH OR FRAUDULENT INTENT OF THE LIABLE PARTY; AND (E) A BREACH BY EITHER PARTY OF ITS REPRESENTATIONS AND WARRANTIES IN ARTICLE 14, IN NO EVENT SHALL EITHER PARTY, ITS AFFILIATES, OR THEIR RESPECTIVE DIRECTORS, OFFICERS, EMPLOYEES OR AGENTS BE LIABLE TO THE OTHER PARTY FOR ANY INDIRECT, INCIDENTAL, PUNITIVE DAMAGES FOR PATENT INFRINGEMENT OR CONSEQUENTIAL DAMAGES (INCLUDING LOST PROFITS OR SALES), WHETHER BASED UPON A CLAIM OR ACTION OF CONTRACT, WARRANTY, NEGLIGENCE, STRICT LIABILITY OR TORT, OR ANY OTHER THEORY OF LAW, ARISING OUT OF THIS AGREEMENT. SUBJECT TO THE EXCEPTION IN THE PRECEDING SENTENCE FOR PUNITIVE DAMAGES FOR PATENT INFRINGEMENT, IN NO EVENT, TO THE MAXIMUM EXTENT PERMITTED UNDER APPLICABLE LAW, SHALL EITHER PARTY, ITS AFFILIATES, OR THEIR RESPECTIVE DIRECTORS, OFFICERS, EMPLOYEES OR AGENTS BE LIABLE TO THE OTHER PARTY FOR ANY SPECIAL, PUNITIVE OR EXEMPLARY DAMAGES, WHETHER BASED UPON A CLAIM OR ACTION OF CONTRACT, WARRANTY, NEGLIGENCE, STRICT LIABILITY OR TORT, OR ANY OTHER THEORY OF LAW, ARISING OUT OF THIS AGREEMENT.

**15.6 Insurance.** BMS shall maintain a program of self-insurance sufficient to fulfill its obligations under this Agreement at all times during the Term. uniQure shall procure and maintain insurance with respect to its Research Program activities which are consistent with normal business practices of prudent companies similarly situated to uniQure at all times during the Research Term and with respect to its Development and manufacturing activities which are consistent with normal business practices of prudent companies similarly situated to uniQure at all times during the Term. It is understood that such insurance shall not be construed to create a limit of either Party’s liability with respect to its indemnification obligations under this Article 15. uniQure shall provide BMS with written evidence of such insurance upon request. uniQure shall provide BMS with written notice at least thirty (30) days prior to the cancellation, non-renewal or material change in such insurance.

## **16. DISPUTE RESOLUTION**

**16.1 Disputes; Resolution by Executive Officers.** The Parties recognize that disputes as to certain matters may from time to time arise during the Term that relate to decisions to be made by the Parties herein or to the Parties’ respective rights or obligations hereunder. It is the desire of the Parties to establish procedures to facilitate the resolution of disputes arising under this Agreement in an expedient manner by mutual cooperation and without resort to arbitration or litigation. To accomplish this objective, the Parties agree to follow the procedures set forth in this Article 16 if and when a dispute arises under this Agreement, subject to Section 16.5. Accordingly, any disputes, controversies or differences concerning the validity, interpretation or construction of, compliance with, or breach of this Agreement, including any dispute with respect to whether either Party is entitled to terminate this Agreement, in whole or in part (e.g., as to any country, Target, Therapeutic or Product), other than a matter within the final decision-making authority of BMS under Section 2.1(d) (a “**Dispute**”), shall be promptly presented to the Alliance Managers for resolution. If the Alliance Managers are unable to resolve such Dispute within twenty (20) Business Days after such Dispute has been presented to them, then upon the request of either Party by written notice, the Parties agree to meet and discuss in good faith a possible resolution thereof, which good faith efforts shall include at least one in-person meeting between the Executive Officers of each Party within twenty (20) Business Days after receipt by the other Party of such written notice. If such Dispute is not resolved within twenty (20) Business Days following presentation to the Executive Officers, then either Party may invoke the provisions of Section 16.2.

**16.2 Arbitration.** Any Dispute that is not resolved pursuant to Section 16.1, shall be settled by binding arbitration to be conducted as set forth below in this Section 16.2.

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(a) Either Party, following the end of the twenty (20) Business Day period referenced in Section 16.1, may refer such Dispute to arbitration by submitting a written notice of such request to the other Party. In any proceeding under this Section 16.2, there shall be one (1) arbitrator to be agreed to by the Parties, *provided however*, that if the Parties do not agree on the selection of such arbitrator within twenty-one (21) days after delivery of such notice, then there shall be three (3) arbitrators, with each Party nominating one (1) arbitrator within such twenty-one (21) day period in accordance with the then current AAA Rules, and with the two (2) arbitrators so nominated nominating a third arbitrator to serve as chair of the arbitration tribunal, with such nomination of the third arbitrator to be made within twenty (20) days after the selection of the first two (2) arbitrators. The arbitrator(s) shall be neutral and independent of both Parties and all of their respective Affiliates, shall have significant experience and expertise in licensing and partnering agreements in the pharmaceutical and biotechnology industries, shall have appropriate experience with respect to the matter(s) to be arbitrated, and shall have some experience in mediating or arbitrating issues relating to such agreements. In the case of any Dispute involving an alleged failure to use Diligent Efforts, the arbitrator(s) shall in addition be an individual with experience and expertise in the worldwide development and commercialization of pharmaceuticals and the business, legal and scientific considerations related thereto. In the case of a Dispute involving a scientific or accounting matter or determination, an Expert having applicable expertise and experience will be selected by the Parties to assist the arbitrator(s) in such scientific or accounting matter or determination (and the arbitrator(s) will select such Expert if the Parties cannot agree on such Expert within twenty (20) days following the selection of the arbitrator(s)). The governing law in Section 17.9 shall govern such proceedings. No individual will be appointed to arbitrate a dispute pursuant to this Agreement unless he or she agrees in writing to be bound by the provisions of this Section 16.2. The place of arbitration will be New York, New York, U.S. unless otherwise agreed to by the Parties, and the arbitration shall be conducted in English.

(b) The arbitrator(s) shall set a date for a hearing that shall be held no later than sixty (60) days following the appointment of the arbitrator(s). The Parties shall have the right to be represented by counsel. Except as provided herein, the arbitration shall be administered and finally resolved through arbitration administered by the American Arbitration Association (the “**AAA**”) under its International Arbitration Rules (the “**AAA Rules**”) applicable at the time of the notice of arbitration pursuant to Section 16.2(a).

(c) The arbitrator(s) shall use reasonable efforts to rule on each disputed issue within thirty (30) days after completion of the hearing described in Section 16.2(b). All rulings of the arbitrator(s) shall be in writing and shall be delivered to the Parties as soon as is reasonably possible. The arbitrator(s) shall render a “reasoned decision” which shall include findings of fact and conclusions of law. Any arbitration award may be entered in and enforced by a court in accordance with Section 16.3.

**16.3 Award.** Any award to be paid by one Party to the other Party as determined by the arbitrator(s) as set forth above under Section 16.2 shall be promptly paid in Dollar free of any tax, deduction or offset; and any costs, fees or taxes incident to enforcing the award shall, to the maximum extent permitted by Applicable Law, be charged against the Party resisting enforcement. Each Party agrees to abide by the award rendered in any arbitration conducted pursuant to this Article 16, and agrees that the award of the arbitrator(s) shall be final and binding on each Party and its respective successors and assigns, judgment may be entered thereupon and enforced in any court of competent jurisdiction pursuant to the United Nations Convention on the Recognition and Enforcement of Foreign Arbitral Awards or other Applicable Law and other courts may award full faith and credit to such judgment in order to enforce such award. With respect to money damages, nothing contained herein shall be construed to permit the arbitrator(s) or any court or any other forum to award any damages not permitted under Section 15.5. By entering into this agreement to arbitrate, the Parties expressly waive any claim for damages not permitted under Section 15.5.

**16.4 Costs.** The costs in connection with any arbitration procedure, consisting of (a) the reasonable legal fees of each Party, (b) the fees of engaging the arbitrator(s) (and any Expert engaged by the

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arbitrators) and (c) payments to the AAA for the conduct of the arbitration, shall be allocated to each Party pro rata to such Party’s success or failure in such arbitration procedure. For clarity, if a Party fully succeeds in such arbitration procedure, the other Party shall pay (x) its own legal fees in connection with such arbitration procedure, (y) all reasonable legal fees of the succeeding Party in connection with such arbitration procedure, and (z) all fees of engaging the arbitrator(s) (and any Expert engaged by the arbitrators) and payments to the AAA for the conduct of the arbitration.

**16.5 Injunctive Relief.** Nothing in this Article 16 will preclude either Party from seeking equitable relief or interim or provisional relief from a court of competent jurisdiction, including a temporary restraining order, preliminary injunction or other interim equitable relief, concerning a Dispute either prior to or during any arbitration if necessary to protect the interests of such Party or to preserve the status quo pending the arbitration proceeding. For the avoidance of doubt, nothing in this Section 16.5 shall otherwise limit a breaching Party’s opportunity to cure a material breach as permitted in accordance with Section 13.3 or Section 13.4.

**16.6 Confidentiality.** The arbitration proceedings shall be confidential and the arbitrator(s) shall issue appropriate protective orders to safeguard each Party’s Confidential Information. Except as required by Applicable Law, no Party shall make (or instruct the arbitrator(s) to make) any public announcement with respect to the arbitration proceedings or decision of the arbitrator(s) without prior written consent of the other Party. The existence of any Dispute submitted to arbitration, and any award, shall be kept in confidence by the Parties and the arbitrator(s), except as required in connection with the enforcement of such award or as otherwise required by Applicable Law.

**16.7 Survivability.** Any duty to arbitrate under this Agreement shall remain in effect and be enforceable after termination of this Agreement for any reason.

## **17. MISCELLANEOUS**

**17.1 Entire Agreement; Amendments.** This Agreement, including the Exhibits hereto and each Certificate pursuant to this Agreement (which Exhibits and Certificates are incorporated into and made a part of this Agreement), sets forth the complete, final and exclusive agreement and all the covenants, promises, agreements, warranties, representations, conditions and understandings between the Parties hereto with respect to the subject matter hereof and supersedes, as of the Effective Date, all prior agreements and understandings between the Parties with respect to the subject matter hereof, including the Prior CDA (but only with respect to Confidential Information (as that term is defined in the Prior CDA) exchanged between the Parties and their Affiliates under the Prior CDA with respect to uniQure’s heart failure program referred to in Schedule A of the Prior CDA) which shall be dealt with as set forth in Section 12.7. In the event of any inconsistency between the Research Plan and this Agreement, the terms of this Agreement shall prevail. There are no covenants, promises, agreements, warranties, representations, conditions or understandings, either oral or written, between the Parties or their respective Affiliates with respect to the subject matter hereof other than as are set forth herein and therein. No subsequent alteration, amendment, change or addition to this Agreement shall be binding upon the Parties unless reduced to writing and signed by an authorized representative of each Party.

**17.2 Export Control.** This Agreement is made subject to any restrictions concerning the export of products or technical information from the U.S. or other countries which may be imposed upon or related to uniQure or BMS from time to time. Each Party agrees that it shall not export, directly or indirectly, any technical information acquired from the other Party under this Agreement or any products using such technical information to a location or in a manner that at the time of export requires an export license or other governmental approval, without first obtaining the written consent to do so from the appropriate Governmental Authority.

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### **17.3 Rights in Bankruptcy.**

(a) All rights and licenses granted under or pursuant to this Agreement by one Party to the other are, for all purposes of Section 365(n) of Title 11 of the United States Code (“**Title 11**”), licenses of rights to “intellectual property” as defined in Title 11, and, in the event that a case under Title 11 is commenced by or against either Party (the “**Bankrupt Party**”), the other Party shall have all of the rights set forth in Section 365(n) of Title 11 to the maximum extent permitted thereby. During the Term, each Party shall create and maintain current copies to the extent practicable of all such intellectual property. Without limiting the Parties’ rights under Section 365(n) of Title 11, if a case under Title 11 is commenced by or against the Bankrupt Party, the other Party shall be entitled to a copy of any and all such intellectual property and all embodiments of such intellectual property, and the same, if not in the possession of such other Party, shall be promptly delivered to it (i) before this Agreement is rejected by or on behalf of the Bankrupt Party, within thirty (30) days after the other Party’s written request, unless the Bankrupt Party, or its trustee or receiver, elects within thirty (30) days to continue to perform all of its obligations under this Agreement, or (ii) after any rejection of this Agreement by or on behalf of the Bankrupt Party, if not previously delivered as

provided under clause (i) above. All rights of the Parties under this Section 17.3 and under Section 365(n) of Title 11 are in addition to and not in substitution of any and all other rights, powers, and remedies that each Party may have under this Agreement, Title 11, and any other Applicable Law. The non-Bankrupt Party shall have the right to perform the obligations of the Bankrupt Party hereunder with respect to such intellectual property, but neither such provision nor such performance by the non-Bankrupt Party shall release the Bankrupt Party from any such obligation or liability for failing to perform it.

(b) The Parties agree that they intend the foregoing non-Bankrupt Party rights to extend to the maximum extent permitted by Applicable Law and any provisions of applicable contracts with Third Parties, including for purposes of Title 11, (i) the right of access to any intellectual property (including all embodiments thereof) of the Bankrupt Party or any Third Party with whom the Bankrupt Party contracts to perform an obligation of the Bankrupt Party under this Agreement, and, in the case of the Third Party, which is necessary for the Development, Regulatory Approval, manufacture and Commercialization of Therapeutics and Products and (ii) the right to contract directly with any Third Party described in (i) in this sentence to complete the contracted work.

(c) Any intellectual property provided pursuant to the provisions of this Section 17.3 shall be subject to the licenses and limitations set forth elsewhere in this Agreement and the payment obligations of this Agreement, which shall be deemed to be royalties for purposes of Title 11.

(d) In the event that after the Effective Date uniQure enters into a license agreement with a Third Party with respect to intellectual property that will be sublicensed to BMS hereunder, uniQure will use reasonable efforts to enable BMS to receive a direct license from any such Third Party in the event that such license agreement between uniQure and such Third Party is terminated during the Term solely on account of uniQure becoming a Bankrupt Party.

(e) Notwithstanding anything to the contrary in Article 9, in the event that uniQure is the Bankrupt Party, BMS may take appropriate actions in connection with the Prosecution and Enforcement of any uniQure Patents licensed or assigned to BMS under this Agreement without being required to consult with uniQure before taking any such actions; *provided however*, that such actions are consistent with this Agreement.

(f) The Parties acknowledge that the rights of the non-Bankrupt Party pursuant to this Section 17.3 are subject to, and may be limited (partially or entirely) by any mandatory applicable insolvency laws to which the Bankrupt Party may be subject under local Applicable Law.

**17.4 Force Majeure.** Each Party shall be excused from the performance of its obligations under this Agreement to the extent that such performance is prevented by force majeure (defined below) and the nonperforming Party promptly provides notice of such prevention to the other Party. Such excuse shall be

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continued so long as the condition constituting force majeure continues. The Party affected by such force majeure also shall notify the other Party of the anticipated duration of such force majeure, any actions being taken to avoid or minimize its effect after such occurrence, and shall take reasonable efforts to remove the condition constituting such force majeure. For purposes of this Agreement, “force majeure” shall include conditions beyond the control of the Parties, including an act of God, acts of terrorism, enactment of any mandatory Applicable Law after the Signing Date prohibiting the nonperforming Party to perform its obligations under this Agreement, war, acts of war (whether war be declared or not), labor strike or lock-out, civil commotion, epidemic, failure or default of public utilities or common carriers, destruction of production facilities or materials by fire, earthquake, storm or like catastrophe. The payment of invoices due and owing hereunder shall in no event be delayed by the payer because of a force majeure affecting the payer.

**17.5 Notices.** Any notice required or permitted to be given under this Agreement shall be in writing, shall specifically refer to this Agreement, and shall be provided by hand, by first class certified or registered mail, postage prepaid, return receipt requested, or by a reputable international expedited delivery service to the other Party at the address or such other address as may be specified by such Party in writing in accordance with this Section 17.5, and shall be deemed to have been given for all purposes (a) when received, if hand-delivered or sent by a reputable international expedited delivery service, or (b) five (5) Business Days after mailing, if mailed by first class certified or registered mail, postage prepaid, return receipt requested.

For uniQure: uniQure Biopharma B.V.  
P.O. Box 22506  
1100 DA Amsterdam  
The Netherlands  
Attention: CEO  
Facsimile: +31 20 566 9272

With a copy to: Dechert LLP  
Tower 185  
Friedrich-Ebert-Anlage 35-37  
60327 Frankfurt am Main  
Germany  
Attention: Dr. Rüdiger Herrmann  
Facsimile: +49 69 7706 19 19

For BMS: Bristol-Myers Squibb Company  
Route 206 and Province Line Road  
Princeton, NJ 08543-4000  
USA  
Attention: Senior Vice President, Strategy, Alliances and Transactions

With a copy to: Bristol-Myers Squibb Company  
Route 206 and Province Line Road  
Princeton, NJ 08543-4000  
USA  
Attention: Vice President and Assistant General Counsel, Business Development and Licensing



**17.6 Independent Contractors.** Each Party shall act solely as an independent contractor, and nothing in this Agreement shall be construed to give either Party the power or authority to act for, bind, or commit the other Party in any way. Nothing herein shall be construed to create the relationship of partners, principal and agent, or joint-venture partners between the Parties.

**17.7 Maintenance of Records.** Each Party shall maintain complete and accurate records of all work conducted under this Agreement and all results, data and developments made pursuant to its efforts under this Agreement. Such records shall be complete and accurate and shall fully and properly reflect all work done and results achieved in the performance of this Agreement in sufficient detail and in good scientific manner appropriate for patent and regulatory purposes. Each Party shall maintain such records for a period of four (4) years after such records are created; *provided however*, that records may be maintained for an appropriate longer period in accordance with each Party's internal policies on record retention in order to ensure the preservation, prosecution, maintenance or enforcement of intellectual property rights. Each Party shall keep and maintain all records required by Applicable Law with respect to Therapeutics and Products.

**17.8 Assignment; Change of Control.**

(a) Neither Party may assign this Agreement, or assign or transfer any rights or obligations hereunder, without the prior written consent of the other, except that a Party may make such an assignment or transfer without the other Party's consent, unless where such assignment or transfer requires the prior consent of any Third Party licensor of uniQure (i) to any Affiliate of such Party, provided that such assignment or transfer shall not adversely affect the other Party's rights and obligations under this Agreement and that such assigning/transferring Party remains jointly and severally liable with such Affiliate for the performance of this Agreement or the transferred or assigned obligations, or (ii) to any Third Party successor-in-interest or purchaser of all or substantially all of the business or assets of such Party to which this Agreement relates (with such business and assets, in the case of uniQure, to include the uniQure Technology and personnel with requisite expertise necessary to perform uniQure's obligations under this Agreement, including conducting the activities assigned to uniQure under the Research Program), whether in a merger, combination, reorganization, sale of stock, sale of assets or other transaction; *provided however*, that in case of (ii) the assigning/transferring Party provides written notice to the other Party of such assignment or transfer and the assignee shall have agreed in writing to be bound (or is otherwise required by operation of Applicable Law to be bound) in the same manner as such assigning Party hereunder. **Exhibit S** contains a list of Third Party agreements as of the Effective Date where the prior consent of Third Party Licensors is required for the assignment of this Agreement or the assignment or transfer of any right or obligation hereunder. uniQure shall update Exhibit S from time to time by providing written notice to BMS to add Third Party agreements that require such Third Party licensor consent. If a Third Party agreement is not listed in Exhibit S, then BMS shall have no obligation to seek the prior consent of uniQure. Any permitted assignment or transfer shall be binding on the successors of the assigning/transferring Party. Any assignment or transfer or attempted assignment or transfer by either Party in violation of the terms of this Section 17.8 shall be null, void and of no legal effect. For clarity, the provisions of this Section 17.8 shall not apply to or encompass sublicensing of the rights licensed to a Party under this Agreement.

(b) In the event that uniQure is acquired in a Change of Control Transaction by a Third Party (such Third Party, hereinafter referred to as an "**Acquirer**"), then the intellectual property of such Acquirer held or developed by such Acquirer prior to or after such acquisition (other than intellectual property developed by such Acquirer in the course of conducting uniQure's activities under this Agreement) shall be excluded from the uniQure Technology, and such Acquirer (and Affiliates of such Acquirer which are not controlled by (as defined under the Affiliate definition in Article 1) uniQure itself) shall be excluded from the Affiliate definition solely for purposes of the applicable components of the uniQure Technology. For clarity, any intellectual property developed by the Acquirer in the course of conducting uniQure's activities under this Agreement shall be included within the uniQure Technology to the extent such

intellectual property would have been so included had it been developed by uniQure. For further clarity, the Acquirer has sole discretion as to whether it will contribute its intellectual property or know-how to uniQure's activities and uniQure Technology under this Agreement.

(c) In the event that BMS is acquired in a Change of Control Transaction by an Acquirer, then the intellectual property of such Acquirer held or developed by such Acquirer prior to or after such acquisition (other than intellectual property developed by such Acquirer in the course of conducting BMS' activities under this Agreement) shall be excluded from the licenses to intellectual property rights of BMS hereunder, and such Acquirer (and Affiliates of such Acquirer which are not controlled by (as defined under the Affiliate definition in Article 1) BMS itself) shall be excluded from the Affiliate definition solely for purposes of the applicable components of intellectual property rights of BMS. For clarity, any intellectual property developed by the Acquirer in the course of conducting BMS' activities under this Agreement shall be included within the licenses to intellectual property rights of BMS hereunder to the extent such intellectual property would have been so included had it been developed by BMS. For further clarity, the Acquirer has sole discretion as to whether it will contribute its intellectual property or know-how to BMS' activities under this Agreement. Notwithstanding the foregoing, in the event that BMS is acquired in a Change of Control Transaction by an Acquirer, BMS shall procure that BMS (only if BMS is a surviving entity) or Acquirer confirms to uniQure in writing within six (6) months following the effective date of such Change of Control Transaction that BMS (only if BMS is a surviving entity) or Acquirer is willing to support the then-current Research Plan and Development Plan in essentially the same manner as agreed before between BMS and uniQure. If (i) uniQure does not obtain such written confirmation by BMS (only if BMS is a surviving entity) or Acquirer within such six (6) months period, the Alliance Manager at uniQure has provided written notice to the Alliance Manager at BMS (only if BMS is a surviving entity) or Acquirer that it did not receive such confirmation and that that failure to provide such confirmation within thirty (30) days may result in the termination of this Agreement on a Collaboration Target-by-Collaboration Target basis, and the Alliance Manager at BMS (only if BMS is a surviving entity) or Acquirer does not provide such confirmation within thirty (30) days of receipt of such written notice or (ii) Acquirer decides not to continue research and Development of any Therapeutic or Product for particular Collaboration Targets in accordance with the then-current Research Plan and Development Plan, then uniQure may terminate this Agreement on a Collaboration Target-by-Collaboration Target basis for those Collaboration Targets for which the Alliance Manager at BMS (only if BMS is a surviving entity) or Acquirer has not provided written confirmation of BMS' (only if BMS is a surviving entity) or Acquirer's intent to continue to do research and Development for any Therapeutic or Product in accordance with the then-current Research Plan and Development Plan in the case of (i), and those Collaboration Targets for which BMS (only if BMS is a surviving entity) or Acquirer has decided not to continue research and Development for any Therapeutic or Product in accordance with the then-current Research Plan and Development Plan in the case of (ii) upon three (3) months prior written notice to BMS, and Section 13.7, except for Section 13.7(h), shall apply.

**17.9 Governing Law.** This Agreement shall be governed by and construed and enforced under the substantive laws of the State of New York (U.S.), excluding any conflicts or choice of law rule or principle that might otherwise make this Agreement subject to the substantive law of another jurisdiction and to the express exclusion of the United Nations Conventions on Contracts for the International Sale of Goods (CISG). For clarification, any dispute relating to the inventorship, scope, validity, enforceability or infringement of any Patent shall be governed by and construed and enforced in accordance with the patent laws of the applicable jurisdiction.

**17.10 Performance by Affiliates.** Subject to the terms and conditions of this Agreement, each Party may discharge any obligations and exercise any rights hereunder through any of its Affiliates. Each Party hereby guarantees the performance by its Affiliates of such Party's discharged obligations under this Agreement, and shall cause its Affiliates to comply with the provisions of this Agreement in connection with such performance. Any breach by a Party's Affiliate of any of such Party's obligations under this Agreement shall be deemed a breach by such Party, and the other Party may proceed directly against such Party without

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any obligation to first proceed against such Party's Affiliate.

**17.11 Further Actions.** Each Party agrees to execute, acknowledge and deliver such further instruments, and to do all such other acts, as may be necessary or appropriate in order to carry out the purposes and intent of this Agreement.

**17.12 Compliance with Applicable Law.** Each Party shall comply with Applicable Law in the course of performing its obligations or exercising its rights pursuant to this Agreement. Neither Party (nor any of their Affiliates) shall be required under this Agreement to take any action or to omit to take any action otherwise required to be taken or omitted by it under this Agreement if the taking or omitting of such action, as the case may be, could in its reasonable opinion violate any settlement, consent order, corporate integrity agreement, or judgment to which it may be subject from time to time during the Term. Notwithstanding anything to the contrary in this Agreement, neither Party nor any of its Affiliates shall be required to take, or shall be penalized for not taking, any action that such Party reasonably believes is not in compliance with Applicable Law.

**17.13 Severability.** If any one or more of the provisions of this Agreement are held to be invalid or unenforceable by an arbitrator or any court of competent jurisdiction from which no appeal can be or is taken, the provision shall be considered severed from this Agreement and shall not serve to invalidate any remaining provisions hereof. The Parties shall make a good faith effort to replace any invalid or unenforceable provision with a valid and enforceable one such that the objectives contemplated by the Parties when entering this Agreement may be realized to the maximum extent possible.

**17.14 No Waiver.** Neither Party may waive or release any of its rights or interests in this Agreement except in writing. The failure of either Party to assert a right hereunder or to insist upon compliance with any term or condition of this Agreement shall not constitute a waiver of that right or excuse a similar subsequent failure to perform any such term or condition. No waiver by either Party of any condition or term in any one or more instances shall be construed as a continuing waiver of such condition or term or of another condition or term.

**17.15 Interpretation.**

(a) The captions and headings to this Agreement are for convenience only, and are to be of no force or effect in construing or interpreting any of the provisions of this Agreement. Unless specified to the contrary, references to Articles, Sections or Exhibits mean the particular Articles, Sections or Exhibits of this Agreement and references to this Agreement include all Exhibits hereto. Unless the context otherwise clearly requires, whenever used in this Agreement: (a) the words "include", "includes" or "including" shall be construed as incorporating also the phrase "but not limited to" or "without limitation"; (b) the word "day" or "quarter" shall mean a calendar day or quarter, unless otherwise specified; (c) the word "notice" shall mean notice in writing (whether or not specifically stated) and shall include notices, consents, approvals and other written communications contemplated under this Agreement; (d) the words "hereof", "herein", "hereby" and derivative or similar words refer to this Agreement (including any Exhibits); (e) provisions that require that a Party, the Parties or the JSC hereunder "agree", "consent" or "approve" or the like shall require that such agreement, consent or approval be specific and in writing, whether by written agreement, letter, approved minutes or otherwise; (f) words of any gender include the other gender; (g) words using the singular or plural number also include the plural or singular number, respectively; (h) references to any specific law, rule or regulation, or article, section or other division thereof, shall be deemed to include the then-current amendments thereto or any replacement law, rule or regulation thereof; (i) the word "will" shall be construed to have the same meaning and effect as the word "shall"; and (j) the term "and/or" shall be construed such that the phrase "X and/or Y" means "X or Y, or both X and Y". Ambiguities, if any, in this Agreement shall not be construed against any Party, irrespective of which Party may be deemed to have authored the ambiguous provision. The language of this Agreement shall be deemed to be the language

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mutually chosen by the Parties and no rule of strict construction shall be applied against either Party hereto. This Agreement should be interpreted in its entirety and the fact that certain provisions of this Agreement may be cross-referenced in a Section shall not be deemed or construed to limit the application of other provisions of this Agreement to such Section and vice versa.

(b) As used in this Agreement, the phrase 'with respect to a given Collaboration Target' or 'with respect to any Collaboration Target' or 'for a Collaboration Target' (or similar phrases) when referring to BMS' licenses or license rights hereunder (or when referring to the termination of BMS' licenses or license rights hereunder) refers to the licensed uniQure Technology that applies to Therapeutics and Products targeting such Collaboration Target.

**17.16 HSR Filing.**

(a) The Parties shall each as promptly as practicable after the Signing Date, file or cause to be filed with the U.S. Federal Trade Commission ("FTC") and the U.S. Department of Justice ("DOJ") and any relevant foreign Governmental Authority any required filings with respect to the transactions contemplated hereby (each a "Required Filing"); *provided however*, that the Parties shall each file the notifications required to be filed under the HSR Act no later than ten (10) Business Days after the Signing Date. Each Party shall be responsible for its own costs in connection with such filing, except that BMS shall be solely responsible for all applicable filing fees. The Parties shall use commercially reasonable best efforts to respond promptly to any and all requests for additional information made by any of such Government Authorities, and to take or cause to be taken all actions, and do or cause to be done

all things, reasonably necessary, proper or advisable on its part under this Agreement and Applicable Laws to satisfy the conditions set forth in Article 15 and to consummate the transactions contemplated by this Agreement as soon as practicable, including by seeking early termination of the HSR waiting period.

(b) The Parties will, in connection with any Required Filing, (i) reasonably cooperate with each other in connection with any communication, filing or submission and in connection with any investigation or other inquiry, including any proceeding initiated by a private party; (ii) keep the other Party and/or its counsel informed of any communication received by such Party from, or given by such Party to, the FTC, the DOJ or any other U.S. or other Governmental Authority and of any communication received or given in connection with any proceeding by a private party, in each case regarding the transactions contemplated by this Agreement; (iii) consult with each other in advance of any meeting or conference with the FTC, the DOJ or any other Governmental Authority or, in connection with any proceeding by a private party, with any other Person, and to the extent permitted by Applicable Law, give the other Parties and/or their counsel the opportunity to attend and participate in such meetings and conferences; and (iv) permit the other Parties and/or their counsel to review in advance any submission, filing or communication (and documents submitted therewith) intended to be given by it to the FTC, the DOJ or any other Governmental Authority.

(c) The Parties shall use their commercially reasonable best efforts to avoid the entry or enactment of any permanent, preliminary or temporary injunction or other order, decree, decision, determination, judgment, investigation or law that would delay in any material respect, restrain, prevent, enjoin or otherwise prohibit consummation of the transactions contemplated by this Agreement if such action should be reasonably necessary or advisable to avoid, prevent, eliminate or remove the actual, anticipated or threatened (i) commencement of any investigation or proceeding in any forum or (ii) issuance or enactment of any order, decree, decision, determination, judgment or law that would delay in any material respect, restrain, prevent, enjoin or otherwise prohibit consummation of the transactions contemplated hereby by any Governmental Authority or any private party.

**17.17 Financial Transparency.** Each Party acknowledges that the other Party or its Affiliates is subject to Applicable Laws related to the collection and reporting of any payments or transfers of value to

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certain healthcare providers and teaching hospitals (collectively, “**Financial Transparency Laws**”), which include, without limitation, relevant provisions of the Affordable Care Act of 2010 and its implementing regulations for the United States along with similar laws and regulations in other countries. Each Party shall reasonably cooperate with such other Party, at such other Party’s cost for reasonable expenses of the first Party, in the first Party’s compliance with Financial Transparency Laws and promptly provide any information requested by such other Party in connection with this Agreement in a mutually agreed upon format to the extent reasonably necessary (as determined by such other Party) for such other Party to comply with its obligations under the Financial Transparency Laws. Such other Party shall have the right to allocate payments or other transfers of value in connection with this Agreement in any required reporting under Financial Transparency Laws in accordance with its normal business practices.

**17.18 Counterparts.** This Agreement may be executed in counterparts with the same effect as if both Parties had signed the same document, each of which shall be deemed an original, shall be construed together and shall constitute one and the same instrument. This Agreement may be executed and delivered through the email of pdf copies of the executed Agreement.

## **18. CONDITIONS TO CLOSING**

**18.1 Conditions to Obligations of All Parties.** The respective obligation of each party to effect the transactions contemplated by this Agreement shall be subject to the fulfillment or mutual waiver, at or prior to the Effective Date, of each of the following conditions:

(a) **Regulatory Consents.** All filings to be made under the HSR Act or any other similar antitrust Laws (including but not limited to the Netherlands), with respect to the Transaction Agreement and the transactions contemplated hereby and thereby, shall have been made and the applicable waiting period, including all extensions thereof, under the HSR Act or any other similar antitrust Laws (including but not limited to the Netherlands), shall have expired or been terminated.

(b) **No injunction.** No order (whether temporary, preliminary or permanent) by any Governmental Authority of competent jurisdiction prohibiting, restraining, enjoining or rendering illegal the consummation of the transactions contemplated by this Agreement shall have been issued and be continuing in effect, and the consummation of the transactions contemplated by this Agreement shall not have been prohibited or rendered illegal under any Applicable Law.

(c) **Execution of Transaction Agreements.** The Parties shall have executed and delivered this Agreement on the Signing Date and the remaining Transaction Agreements shall have been executed and delivered by uniQure N.V. and BMS on the Signing Date.

*[Signature page follows]*

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**IN WITNESS WHEREOF**, the Parties have caused this Agreement to be executed by their duly authorized representatives on the Signing Date and with effect as of the Effective Date.

**BRISTOL-MYERS SQUIBB COMPANY**

**UNIQURE BIOPHARMA B.V.**

By: \_\_\_\_\_

By: \_\_\_\_\_

Name: \_\_\_\_\_

Name: \_\_\_\_\_



## Exhibit C

## Existing License Agreements

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## Exhibit D

### Summary of the Initial Research Plan as of the Signing Date

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### Gantt Chart for the Lead S100A1 Therapeutic for Gene Therapy

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## Project Budget for the Lead S100A1 Therapeutic for Gene Therapy

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### Gantt Chart for a New Target

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### Project Budget for a New Target

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## Exhibit E

**Collaboration Target as of the Signing Date**
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## Exhibit F

### Initial Members of JSC

For uniQure:

$$[**].$$

**Exhibit G****Form of Certificate****CERTIFICATE FOR COLLABORATION TARGETS**

This Certificate for Collaboration Targets (the “Certificate”) is given pursuant to Section 3.4(b) of the Collaboration and License Agreement executed as of April 6, 2015 (the “Agreement”) by and between **uniQure Biopharma B.V.** (“**uniQure**”) and **Bristol-Myers Squibb Company** (“**BMS**”) effective on [date] (the “Certificate Effective Date”). All capitalized terms not otherwise defined herein shall have the meanings assigned to them in the Agreement, as may be amended from time to time.

The following representations and warranties apply with respect to the proposed Collaboration Target identified in Attachment A attached hereto (the “Selected Target”) and any Therapeutics and Products with respect to such Selected Target as contemplated to be Developed and Commercialized in accordance with the Research Program and the Agreement. The following representations and warranties are subject to the terms and conditions of the Agreement including, but not limited to, Article 16 and Article 17 and the limitations set forth in Section 14.3 and Section 15.5 of the Agreement.

uniQure hereby represents and warrants as of the Certificate Effective Date as follows:

- (a) It is not a party to any agreement that would prevent it from granting the rights granted to BMS under the Agreement or performing its obligations under the Agreement with respect to the Selected Target.
- (b) uniQure has not entered into any agreements, either oral or written, with any Third Party relating to the Development, Commercialization or manufacture of Therapeutics or Products with respect to the Selected Target in the Field in the Territory.
- (c) Except as disclosed by uniQure in writing to BMS’ Patent Contact prior to the Certificate Effective Date, there are no pending, and to uniQure’s knowledge, no threatened, actions, suits or proceedings against uniQure involving the uniQure Technology as it relates to Therapeutics or Products with respect to the Selected Target.
- (d) uniQure has not granted (and uniQure covenants that during the Term it shall not grant, except in accordance with the express terms and conditions of the Agreement) any license or any option for a license under the uniQure Technology to any Third Party to make, use or sell any Therapeutic or Product with respect to the Selected Target in the Field in any country in the Territory.

*[Signature page follows]*

uniQure has caused this Certificate to be executed by its duly authorized representative effective as of the Certificate Effective Date.

**UNIQURE BIOPHARMA B.V.**

By: \_\_\_\_\_

Name: \_\_\_\_\_

Title: \_\_\_\_\_

**Attachment A to Certificate for Collaboration Targets****Selected Target****Exhibit H****Delineation and Interpretation of Subsequent Therapeutics and Back-up Therapeutics**

**Example 1**

**Example 2**

**Example 3**

**Exhibit I**

**Press Release**



**Bristol-Myers Squibb and uniQure Enter into Exclusive Strategic Collaboration to Develop Gene Therapies for Cardiovascular Diseases**

(New York and Amsterdam, the Netherlands, April 6, 2015) — Bristol-Myers Squibb Company (NYSE:BMJ) and uniQure N.V. (NASDAQ:QURE) announced today an agreement that provides Bristol-Myers Squibb with exclusive access to uniQure’s gene therapy technology platform for multiple targets in cardiovascular diseases. The collaboration includes uniQure’s proprietary gene therapy program for congestive heart failure that is intended to restore the heart’s ability to synthesize S100A1, a calcium sensor and master regulator of heart function, and thereby improve clinical outcomes for patients with reduced ejection fraction. Beyond cardiovascular diseases, the agreement also includes the potential for target-exclusive collaboration in other disease areas. In total, the companies may collaborate on ten targets, including S100A1.

uniQure will lead discovery efforts and be responsible for manufacturing of clinical and commercial supplies using its vector technologies and its industrial, proprietary insect-cell based manufacturing platform. Bristol-Myers Squibb will lead development and regulatory activities across all programs and be responsible for all research and development costs. Bristol-Myers Squibb will be solely responsible for commercialization of all products from the collaboration.

“Bristol-Myers Squibb has an excellent and long-standing track record of success in discovering and developing treatments for cardiovascular diseases and in embracing advancing technologies for the treatment of human diseases,” said Carl Decicco, Ph.D., Head of Discovery, R&D, Bristol-Myers Squibb. “Collaborating with uniQure, a clear leader in the field with an innovative and validated gene therapy platform, further strengthens our capability to bring forward transformational new therapeutics for difficult-to-treat diseases, including cardiovascular diseases such as heart failure.”

“Bristol-Myers Squibb’s strength in the cardiovascular area and its commitment to gene therapy will allow them to leverage the full breadth and capacity of our platform for cardiovascular diseases,” said Joern Aldag, Chief Executive Officer of uniQure. “This collaboration will accelerate the application of gene therapy for large patient populations suffering from heart diseases and will complement the further development of uniQure’s internal pipeline in two focus areas: liver diseases, including hemophilia, and CNS, including lysosomal storage diseases.”

S100A1, to be made within three months of the closing and an initial equity investment in uniQure for a number of shares that will equal 4.9% of the total number of shares outstanding following such issuance, at a purchase price of \$33.84 per share, or at least \$32 million in total. Bristol-Myers-Squibb will acquire an additional 5.0% ownership before December 31, 2015, at a 10% premium, and will be granted two warrants to acquire up to an additional 10% equity interest, at a premium, based on additional targets being introduced into the collaboration. The parties have also agreed to enter into a supply contract, under which uniQure will undertake manufacturing of all gene therapy products under the collaboration.

uniQure will be eligible to receive research, development and regulatory milestone payments, including up to \$254 million for the lead S100A1 therapeutic and up to \$217 million for each other gene therapy product potentially developed under the collaboration. uniQure is also eligible to receive net sales based milestone payments and tiered single to double-digit royalties on product sales.

“It is immensely exciting to see the potential of our initial discoveries recognized first by uniQure and then advanced to a stage where we can build a portfolio of gene therapies to treat cardiovascular disease in partnership with Bristol-Myers Squibb,” added Prof. Dr. Patrick Most, Managing Director of uniQure Germany (formerly known as InoCard). “I would like to thank my colleagues in Heidelberg, Amsterdam and Lexington, Massachusetts for the teamwork that has contributed to bringing the lead S100A1 therapeutic closer to helping patients.”

uniQure and Bristol-Myers Squibb anticipate the collaboration to be effective during the second quarter of 2015. The effectiveness of the transaction is subject to customary closing conditions, including clearance under the Hart-Scott-Rodino Antitrust Improvements Act. The initial issuance by uniQure of equity to Bristol-Myers Squibb also is anticipated to close in the second quarter of 2015 and is subject to the approval by the shareholders of uniQure.

### **About Bristol-Myers Squibb**

Bristol-Myers Squibb is a global biopharmaceutical company whose mission is to discover, develop and deliver innovative medicines that help patients prevail over serious diseases. For more information, please visit [www.bms.com](http://www.bms.com) or follow us on Twitter at <http://twitter.com/bmsnews>.

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### **About uniQure**

uniQure is delivering on the promise of gene therapy through single treatments with potentially curative results. We have developed a modular platform to rapidly bring new disease-modifying therapies to patients with severe disorders. We are engaged in multiple partnerships and have obtained regulatory approval of our lead product, Glybera, in the European Union for a subset of patients with LPLD. [www.uniQure.com](http://www.uniQure.com)

### **Bristol-Myers Squibb Forward-Looking Statement**

*This press release contains “forward-looking statements” as that term is defined in the Private Securities Litigation Reform Act of 1995 regarding the research, development and commercialization of pharmaceutical products. Such forward-looking statements are based on current expectations and involve inherent risks and uncertainties, including factors that could delay, divert or change any of them, and could cause actual outcomes and results to differ materially from current expectations. No forward-looking statement can be guaranteed. Among other risks, there can be no guarantee that any of the investigational gene therapy programs described in this release will be successful. Forward-looking statements in this press release should be evaluated together with the many uncertainties that affect Bristol-Myers Squibb’s business, particularly those identified in the cautionary factors discussion in Bristol-Myers Squibb’s Annual Report on Form 10-K for the year ended December 31, 2014 in our Quarterly Reports on Form 10-Q and our Current Reports on Form 8-K. Bristol-Myers Squibb undertakes no obligation to publicly update any forward-looking statement, whether as a result of new information, future events or otherwise.*

### **uniQure Forward-Looking Statement**

*This press release contains forward-looking statements. All statements other than statements of historical fact are forward-looking statements, which are often indicated by terms such as “anticipate,” “believe,” “could,” “estimate,” “expect,” “goal,” “intend,” “look forward to”, “may,” “plan,” “potential,” “predict,” “project,” “should,” “will,” “would” and similar expressions. Forward-looking statements are based on management’s beliefs and assumptions and on information available to management only as of the date of this press release. These forward-looking statements include, but are not limited to, statements regarding the development of gene therapies for cardiovascular disease, the success of our collaboration with Bristol-Myers Squibb, the election by Bristol-Myers Squibb to extend the range of target indications covered by our collaboration, and the risk of cessation, delay or lack of success of any of our ongoing or planned clinical studies and/or development of our product candidates. Our actual results could differ materially from those anticipated in these forward-looking statements for many reasons, including, without limitation, risks associated with collaboration arrangements, our and our collaborators’ clinical development activities, regulatory oversight, product commercialization and intellectual property claims, as well as the risks, uncertainties and other factors described under the heading “Risk Factors” in uniQure’s 2013 Annual Report on Form 20-F filed with the Securities and Exchange Commission on April 25, 2014 and its 2014 Annual Report on Form 20-F to be filed with the Securities and Exchange Commission. Given these risks, uncertainties and other factors, you should not place undue reliance on these forward-looking statements, and we assume no obligation to update these forward-looking statements, even if new information becomes available in the future.*

### **Media:**

Bristol-Myers Squibb  
Sarah Koenig, 609-252-4145, [sarah.koenig@bms.com](mailto:sarah.koenig@bms.com)

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Bristol-Myers Squibb  
Ranya Dajani, 609-252-5330, ranya.dajani@bms.com

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- 1) If uniQure or its relevant Affiliate is not AAALAC accredited or loses its AAALAC accreditation at any time during the term of the Agreement, it will complete an Animal Welfare Risk Assessment questionnaire prior to the commencement (or continuation) of the performance of the Research Program and provide the BMS Veterinary Sciences Department with sufficient documentation in such manner, format and frequency as BMS may require, in its sole reasonable discretion, to assure appropriate care and use of animals. Such documentation may include, without limitation, government inspection reports, animal test methods, animal use protocols, and any other written descriptions of animal care and use. This documentation must demonstrate compliance with [the current Guide for the Care and Use of Laboratory Animals (*Institute for Laboratory Animal Resources, National Research Council, National Academy of Sciences*) or an equivalent reference], and apply to all animal research conducted for BMS under the Research Program by any research facility of uniQure, its Affiliates or any of their (sub)contractors.
- 2) uniQure will comply, and will procure that any relevant Affiliate will comply, with all Applicable Laws governing animal research and the **Harmonized Animal Care and Use Standards for Bristol-Myers Squibb Company Contractors**, set forth below. Accreditation by the Association for Assessment and Accreditation of Laboratory Animal Care International (AAALAC) serves as presumptive evidence of an acceptable animal care and use program. Therefore, additional assurances, other than those set forth in the **Harmonized Animal Care and Use Standards for Bristol-Myers Squibb Company Contractors** related to animal care and environment will not be required from Contractors that are accredited by AAALAC.

- 3) uniQure uses the Academic Medical Center Amsterdam (AMC) for its animal research conducted for BMS under the Research Program, which is AAALAC accredited and therefore, as long as AMC does not lose its AAALAC accreditation, additional assurances, other than those set forth in the **Harmonized Animal Care and Use Standards for Bristol-Myers Squibb Company Contractors** related to animal care and environment will not be required from uniQure under this Agreement.
- 4) Upon reasonable, advance notice during the term of the Agreement, representatives of BMS shall have the right to inspect the research facilities and to audit the care, treatment, and use of the animals used in the research activities under the Research Program. This includes the right to review any correspondence with or reports from Governmental Authorities or accrediting organizations responsible for animal welfare or quality assurance.

### **Harmonized Animal Care & Use Standards for Bristol-Myers Squibb Co. Contractors**

Bristol-Myers Squibb (Company/BMS) is committed to providing safe and effective, quality products. This commitment, at times, requires the responsible care and use of animals for research or testing. It is the policy of the Company to insist that its scientists always consider replacing the use of animals by other methods, reducing the number of animals used, and refining procedures to enhance animal welfare. BMS' Contractors are expected to treat all animals used in Company related research in a caring and humane manner while maintaining a standardized environment and husbandry program.

BMS' Veterinary Sciences Department must evaluate the animal care and use program of a BMS Contractor (commercial laboratories, academic institutions, veterinary practices, farms, and other animal care agencies/facilities (collectively, "**Contractor**")) for conformance to published regulations and BMS' standards for the humane care, treatment, and use of all animals.

The Guide for the Care and Use of Laboratory Animals (*Institute for Laboratory Animal Resources, National Research Council, National Academy of Sciences*), BMS' internal standards, and applicable governmental regulations are BMS' primary standards for conducting animal-based testing. To assist each applicable unit of the Company in assuring appropriate animal care and use for Company-sponsored work performed at AAALAC Accredited Contractor facilities, the following standards apply:

- All research, studies, tests, or other procedures under the Agreement involving the use of live animals must be reviewed and approved by the Contractor's Animal Care and Use Committee ("**ACUC**"), or an equivalent committee (e.g., animal ethics committee), if available.
- Research, studies, tests, or other procedures paid for under the Agreement involving the use of live animals must also be reviewed by the BMS Veterinary Sciences Department for deviations from accepted practices.
- It is the responsibility of the BMS sponsor to inform BMS Veterinary Sciences of the activities before they begin. Such review is not required for donations of product (test material), benevolent grants, or other activities which BMS does not specifically request or direct and activities provided for in the Research Plan.
- Suitable documents for review include, without limitation, protocols, animal test method forms, written descriptions of related animal use activities.
- The Contractor must have written procedures or standards for the humane care and treatment of all animals utilized.

### **Additional Standards for Non-AAALAC Accredited Contract Facilities**

The Contractor must comply with all Applicable Law at the location of the research activities under the Research Plan. Where the local Applicable Law is less stringent than the standards set forth below, the standards below, which comply with the current Guide for the Care and Use of Laboratory Animals (*Institute for Laboratory Animal Resources, National Research Council, National Academy of Sciences*) should be followed for all animal research conducted for BMS. In all instances where Guide recommendations are different from Applicable Law requirements, the higher standard should apply.

Only animals that are lawfully acquired in compliance with Applicable Law shall be used by Contractor. The Research Facility must have written procedures that describe its standards for the humane care and treatment of all animals and provide the necessary resources to ensure adherence with these basic standards.

The following standards will be used to evaluate animal welfare at research facilities.

- Animals must be provided with an environment (housing design, ambient temperature, humidity, lighting, ventilation, etc.) that is consistent with their physiological and behavioral needs. Appropriate sanitation and environmental enrichment should be provided.
- Animals must be provided a diet that matches their nutritional needs, including having an adequate supply of clean, potable water, unless scientifically justified otherwise.
- Animals must be provided veterinary medical care consistent with current veterinary medical standards. This includes routine surveillance for pathogens, preventative care, care for research-related medical conditions, and weekend, holiday, and emergency care. A program to document medical care must be established.
- Animals must be humanely euthanatized using accepted veterinary procedures. Euthanasia methods shall be consistent with the *American Veterinary Medical Association's Guidelines on Euthanasia*, or equivalent references. Animals that would otherwise suffer severe or chronic pain or distress that cannot be relieved must be euthanatized at the end of the procedure or, if appropriate, during the procedure. The ACUC and investigator must weigh the objectives of the study against potential animal welfare concerns (harm-benefit assessment). Humane endpoints should be established and used to determine when euthanasia is appropriate.

- All staff working with animals must have the appropriate training and experience to conduct their assigned duties.
- A pest control program must be in place to prevent vermin infestation.
- The animals selected for a procedure must be the appropriate species, have the appropriate health status, and be the minimum number required to obtain valid results.
- Non-animal methods will be used in place of animal research whenever scientifically appropriate.
- Where exceptions are required to the aforementioned standards, the decision to permit an exception should not rest with the investigator directly concerned, but should be made by an appropriate review group such as an institutional ACUC or animal ethics committee. Such exceptions should not be made solely for the purposes of teaching or demonstration.

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#### **Exhibit L**

##### **Third Party Agreements Requiring Consent to Sublicense and/or Having Sublicensing-Specific Payment Obligations**

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#### **Exhibit M**

##### **Publication Rights of Prof. [\*\*] and Prof. [\*\*]**

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#### **Exhibit N**

##### **Third Party Licenses Cost Sharing**

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#### **Exhibit O**

##### **uniQure Platform Technology Patents**

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#### **Exhibit P**

##### **[\*\*]'s Patents**

(a) [\*\*]

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#### **Exhibit Q**

##### **uniQure Manufacturing Patents**

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**Exhibit R**

**Liens to uniQure Technology**

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**Exhibit S**

**Third Party Agreements Requiring Consent for Assignments**

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

EXECUTION VERSION

## SHARE SUBSCRIPTION AGREEMENT

By and Between

BRISTOL-MYERS SQUIBB COMPANY

and

UNIQUE N.V.

Dated as of April 6, 2015

UNIQUE N.V.

## SHARE SUBSCRIPTION AGREEMENT

THIS SHARE SUBSCRIPTION AGREEMENT (the “**Agreement**”) is made and entered into as of April 6, 2015 (the “**Signing Date**”), by and between uniQure N.V., a public company with limited liability (*naamloze vennootschap*) incorporated under the laws of the Netherlands (the “**Company**”), and Bristol-Myers Squibb Company, a Delaware corporation (the “**Purchaser**”).

WHEREAS, uniQure Biopharma B.V., an Affiliate of the Company, and the Purchaser are entering into that certain Collaboration and License Agreement of even date herewith (the “**Collaboration Agreement**”);

WHEREAS, the obligations in the Collaboration Agreement are conditioned upon the execution and delivery of this Agreement, pursuant to which the Company will issue and sell to the Purchaser a number of its ordinary shares, par value €0.05 per share (the “**Ordinary Shares**”) as provided for herein; and

WHEREAS, the Purchaser desires to purchase, and the Company desires to sell, the Ordinary Shares on the terms and conditions set forth herein;

NOW, THEREFORE, in consideration of the foregoing recitals and the mutual promises, representations, warranties, and covenants hereinafter set forth and for other good and valuable consideration, the receipt and sufficiency of which are hereby acknowledged, the parties hereto agree as follows:

**1. Definitions.** When used in this Agreement, the following terms shall have the respective meanings specified below:

“**Action**” shall mean any action, cause or action, suit, prosecution, investigation, litigation, arbitration, hearing, order, claim, complaint or other proceeding (whether civil, criminal, administrative, investigative or informal) by or before any Governmental Authority or arbitrator.

“**Affiliate**” shall mean, with respect to any Person, another Person which controls, is controlled by or is under common control with such Person. A Person shall be deemed to control another Person if such Person possesses, directly or indirectly, the power to direct or cause the direction of the management and policies of such Person, whether through the ownership of voting securities, by contract or otherwise. Without limiting the generality of the foregoing, a Person shall be deemed to control another Person if any of the following conditions is met: (i) 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 (ii) 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. For the purposes of this Agreement, in no event shall the Purchaser or any of its Affiliates be deemed Affiliates of the Company or any of its Affiliates, nor shall the Company or any of its Affiliates be deemed Affiliates of the Purchaser or any of its Affiliates.

“**AGM BMS Transaction Authorization**” shall mean the approval by the Company’s shareholders at the 2015 Annual General Meeting of the Company’s issuance of Ordinary Shares pursuant to the terms of the Transaction Agreements, in a form and manner compliant with applicable Laws and the NASDAQ listing rules.

“**AGM General Authorization**” shall mean the designation by the 2015 Annual General Meeting of shareholders of the Company of the management board of the Company as the competent body to issue a number of Ordinary Shares at least equal to the maximum number of Ordinary Shares issuable under the Transaction Agreements in the event that the AGM BMS Transaction Authorization is not approved, and to grant rights to subscribe for Ordinary Shares and to limit or exclude pre-emptive rights in connection therewith, subject to the approval of the supervisory board of the Company, in each case in a form and manner compliant with applicable Laws.

“**Business Day**” shall mean any day except Saturday, Sunday and any day on which banking institutions in New York, New York and Amsterdam, the Netherlands, generally are closed as a result of federal, state or local holiday.

“**Code**” shall mean the United States Internal Revenue Code of 1986, as amended.

**“Consent”** shall mean any approval, authorization, consent, license, franchise, Order, registration, notification, permit, certification, clearance, waiver or other confirmation of or by a Governmental Authority or other Person.

**“Contract”** shall mean, with respect to any Person, any written or oral agreement, contract, commitment, indenture, note, bond, loan, license, sublicense, lease, sublease, undertaking, statement of work or other arrangement to which such Person is a party or by which any of its properties or assets are subject.

**“Draft Annual Report”** shall mean the draft Annual Report on Form 20-F of the Company in respect of the year ended December 31, 2014, in the form provided to the Purchaser prior to the date hereof

**“Employee Benefit Plan”** shall mean any “employee benefit plan” (as such term is defined in Section 3(3) of ERISA, whether or not subject to ERISA), any severance, employment, incentive or bonus, retention, change in control, deferred compensation, termination pay, profit sharing, retirement, welfare, post-employment welfare, fringe benefit, vacation or paid time off, equity or equity-based or any other plan, policy, program, agreement, contract or arrangement (i) that is sponsored, maintained, contributed to, or required to be contributed to by the Company or any of its Subsidiaries or under or with respect to which the Company or any of its Subsidiaries has any current or contingent liability or obligation, or (ii) that provides benefits or compensation to any employee, director, or officer of the Company or any of its Subsidiaries or any other person performing services for the Company or any of its Subsidiaries (including any leased employee or individual co-employed by a “professional employer organization”).

**“Environmental Law”** shall mean all national, supra-national, federal, state, local and foreign Laws concerning public health and safety, worker health and safety, pollution or

protection of the environment; including without limitation all those relating to the generation, handling, transportation, treatment, storage, disposal, release, exposure to or cleanup of hazardous materials, substances or wastes, including petroleum, asbestos, polychlorinated biphenyls, asbestos, noise or radiation.

**“ERISA”** shall mean the United States Employee Retirement Income Security Act of 1974, as amended, and the rulings and regulations thereunder.

**“Exchange Act”** shall mean the U.S. Securities Exchange Act of 1934, as amended, and the rules and regulations promulgated thereunder.

**“Governmental Authority”** shall mean any court, agency, authority, department, regulatory body or other instrumentality of any government or country or of any national, federal, state, provincial, regional, county, city or other political subdivision of any such government or country or any supranational organization of which any such country is a member.

**“Health Care Laws”** means all applicable Laws relating to pricing, marketing, promotion, sale, distribution, coverage, or reimbursement of a drug, biological or medical device.

**“Indebtedness”** shall mean, with respect to any Person at any applicable time of determination, without duplication, (i) all liabilities and obligations for borrowed money, (ii) all liabilities and obligations evidenced by bonds, debentures, notes or other similar instruments or debt securities, (iii) all liabilities and obligations under or in respect of swaps, hedges or similar instruments; (iv) all liabilities and obligations in respect of letters of credit and similar instruments, (v) all liabilities and obligations (contingent or otherwise) arising from or in respect of (a) deferred compensation arrangements, (b) pension plans, or (c) amounts payable as a result of the consummation of the transactions contemplated hereby (regardless of whether any additional event, in addition to the consummation of the transactions contemplated hereby, is required to give rise to such liabilities and obligations), (vi) all guaranties in connection with any of the foregoing, and (vii) all accrued interest, prepayment premiums, fees, penalties, expenses or other amounts payable in respect of any of the foregoing.

**“Intellectual Property”** shall mean all intellectual property and other similar proprietary rights in any jurisdiction, including such rights in and to: (i) any patent (including all reissues, divisions, continuations, continuations-in-part and extensions thereof), patent application, patent disclosure or other patent right, (ii) any trademark, service mark, trade name, business name, brand name, slogan, logo, trade dress and all other indicia of origin together with all goodwill associated therewith, and all registrations, applications for registration, and renewals for any of the foregoing, and (iii) any copyright, work of authorship (whether or not copyrightable), design, design registration, database rights, and all registrations, applications for registration, and renewals for any of the foregoing (and including in all website content and software), (iv) any Internet domain names, and (v) any trade secret, confidential information, know-how and inventions, including processes and formulations.

**“Investor Agreement”** shall mean the Investor Agreement dated as of the date hereof between the Company and the Purchaser, in the form attached hereto as Exhibit A.

**“HSR Act”** shall mean the Hart-Scott-Rodino Antitrust Improvements Act of 1976, as amended, and the rules and regulations promulgated thereto.

**“Knowledge”** shall mean actual knowledge after reasonable investigation of Jorn Aldag, Matthew Kapusta, Hans Preusting and Eric Goossens.

**“Law”** or **“Laws”** shall mean all laws, statutes, rules, regulations, orders, judgments, injunctions and/or ordinances of any Governmental Authority.

**“Leased Real Property”** shall mean all leasehold or subleasehold estates and all other rights to use or occupy any land, buildings, structures, improvements, fixtures or other interest in real property held by the Company or any of its Subsidiaries pursuant to any Lease.

**“Leases”** shall mean all leases, subleases, licenses, concessions and other Contracts pursuant to which the Company or any of its Subsidiaries holds any Leased Real Property as tenant, sublease, licensee or concessionaire (including the rights to all security deposits and other amounts and instruments

deposited by or on behalf of the Company and/or and of its Subsidiaries thereunder) and all material amendments, extensions, renewals, guaranties and other agreements with respect thereto.

“**Liens**” shall mean a lien, charge, security interest, encumbrance, right of first refusal, preemptive right or other restriction.

“**Loan Agreement**” shall mean that certain Amended and Restated Loan and Security Agreement, dated June 26, 2014, by and among uniQure Biopharma B.V., uniQure IP B.V., the subsidiaries of uniQure identified on Schedule 1 thereto, uniQure N.V. and Hercules Technology Growth Capital, Inc.

“**Material Adverse Effect**” shall mean any change, event or occurrence (each, an “**Effect**”) that, individually or when taken together with all other Effects, has had or is reasonably likely to have (i) a material adverse effect on the business, condition (financial or other), assets, liabilities or results of operations of the Company and its Subsidiaries, taken as a whole, or (ii) a material adverse effect on the Company’s ability to timely perform its obligations under, or timely consummate any of the transactions contemplated by, the Transaction Agreements, (including the sale of the Shares, in accordance with the terms of this Agreement), except in the case of (i) or (ii) to the extent that any such Effect results from or arises out of: (A) changes occurring after the date hereof in conditions in the Netherlands or global economy or capital or financial markets generally, including changes in interest or exchange rates, (B) changes occurring after the date hereof in general legal, regulatory, political, economic or business conditions or changes International Financing Reporting Standards or interpretations thereof occurring after the date hereof that, in each case, generally affect the biotechnology or biopharmaceutical industries, (C) acts of war, sabotage or terrorism occurring after the date hereof, or any escalation or worsening of any such acts of war, sabotage or terrorism, (D) earthquakes, hurricanes, floods or other natural disasters occurring after the date hereof, or (E) any action expressly required by the Company contemplated by this Agreement or expressly required by the Collaboration Agreement or with the Purchaser’s written consent; provided, however, that any change, event or occurrence referred to in clauses (A)-(D) above shall be taken

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into account in determining whether a Material Adverse Effect has occurred or would be reasonably likely to occur if such change, event or occurrence has had or would be reasonably likely to have a disproportionate effect on the Company and its Subsidiaries compared to other participants in the biotechnology or biopharmaceutical industries.

“**NASDAQ**” shall mean The NASDAQ Stock Market LLC.

“**Order**” shall mean any assessment, award, decision, injunction, judgment, order, ruling, verdict or writ entered, issued, made, or rendered by any court, administrative agency, or other Governmental Authority or by any arbitrator.

“**Permitted Liens**” shall mean (a) mechanics’, materialman’s, workmens’, repairmen’s’, warehousemen’s, supplier’s, vendor’s, carrier’s and other similar Liens arising or incurred in the ordinary course of business by operation of Law securing amounts that are not yet due and payable, (b) Liens for Taxes, assessments and other charges of Governmental Authorities not yet due and payable, (c) Liens arising under original purchase price conditional sales Contracts and equipment leases with third parties, (d) pledges or deposits to secure obligations under workers or unemployment compensation Laws or to secure other statutory obligations, (e) easements, covenants, conditions and restrictions of record affecting title to the Leased Real Property which do not or would not materially impair the use or occupancy of any Leased Real Property in the operation of the business conducted thereon as of the date of this Agreement, and (f) any zoning, or other governmentally established restrictions of encumbrances.

“**Person**” shall mean any individual, partnership, limited liability company, firm, corporation, trust, unincorporated organization, government or any department or agency thereof or other entity, as well as any syndicate or group that would be deemed to be a Person under Section 13(d)(3) of the Exchange Act.

“**SEC**” shall mean the U.S. Securities and Exchange Commission.

“**Securities Act**” shall mean the U.S. Securities Act of 1933, as amended, and the rules and regulations promulgated thereunder.

“**Tax**” or “**Taxes**” shall mean (i) any federal, state, local, or non-U.S. income, gross receipts, license, payroll, employment, excise, severance, stamp, occupation, premium, windfall profits, environmental (including taxes under Section 59A of the Code), customs duties, capital stock, franchise, profits, withholding, social security (or similar), unemployment, disability, real property, personal property, sales, use, transfer, unclaimed property or escheat (or similar), registration, value added, alternative or add-on minimum, estimated, or other tax of any kind whatsoever, including any interest, penalty, or addition thereto, whether disputed or not; (ii) any liability for or in respect of the payment of any amount of a type described in clause (i) of this definition as a result of being a member of an affiliated, combined, consolidated, unitary or other group for Tax purposes; or (iii) any liability for or in respect of the payment of any amount described in clauses (i) or (ii) of this definition as a transferee or successor, by Contract or otherwise.

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“**Tax Return**” shall mean any return, declaration, report, claim for refund, or information return or statement relating to Taxes, including any schedule or attachment thereto, and including any amendment thereof.

“**Third Party**” shall mean any Person (other than a Governmental Authority) other than the Purchaser, the Company or any Affiliate of the Purchaser or the Company.

“**Trading Day**” shall mean a day on which the Trading Market is open for trading.

“**Trading Market**” shall mean the NASDAQ Global Select Market or New York Stock Exchange to the extent that the Ordinary Shares are then listed on such exchange, as applicable.

“**Transaction Agreements**” shall mean this Agreement, the Investor Agreement and the Warrant Agreements.

“**VWAP**” shall mean, for any date, the price determined by the first of the following clauses that applies: (a) if the Ordinary Shares are then listed or quoted on a Trading Market, the daily volume weighted average price of the Ordinary Shares for such date (or the nearest preceding date) on the Trading Market on which the Ordinary Shares are then listed or quoted as reported by Bloomberg L.P. (based on a Trading Day from 9:30 a.m. (New York City time) to 4:02 p.m. (New York City time)), or (b) in all other cases, the fair market value of a share of Ordinary Shares as determined by an independent appraiser selected in good faith and mutually agreed upon between the Company and the Purchaser, the fees and expenses of which shall be paid by the Company.

“**WARN Act**” shall mean the Worker Adjustment and Retraining Notification Act of 1988, as amended and any similar or related Law.

“**Warrant Agreements**” shall mean each of the Seventh Target Warrant Agreement and the Tenth Target Warrant Agreement, each dated as of the date hereof.

## **2. Closing, Delivery and Payment.**

### **2.1 Closings.**

(a) Subject to the terms and conditions hereof, and in reliance on the representations, warranties, covenants and other agreements hereinafter set forth, at the Initial Closing (as hereinafter defined), the Company hereby agrees to issue to the Purchaser, and the Purchaser agrees to subscribe for, such number of Ordinary Shares as will equal 4.9% of the total number of Ordinary Shares outstanding immediately following such issuance (the “**Initial Shares**”), at a purchase price of \$33.84 per Ordinary Share (the “**Initial Closing**”), free and clean of all Liens. The Initial Closing shall take place on the date two Business Days following receipt of the AGM General Authorization or such other date as may be mutually agreeable to the Company and the Purchaser, at the offices of Kirkland & Ellis LLP, 601 Lexington Avenue, New York, NY 10022 (which time and place are designated as the “**Initial Closing Date**”).

(b) Subject to (i) the terms and conditions hereof, (ii) the payment by the Purchaser to the Company of the Target Designation Fees associated with the first [\*\*] New

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Targets (with such terms as defined in the Collaboration Agreement), and (iii) the Second Closing (as defined below) occurring no later than the Purchase Termination Date (as defined below), at a time to be decided in the Purchaser’s sole discretion that is at least thirty (30) days after the Initial Closing Date but prior to December 31, 2015 (the “**Purchase Termination Date**”), and in reliance on the representations, warranties, covenants and other agreements hereinafter set forth, the Company hereby agrees to issue to the Purchaser, and the Purchaser agrees to subscribe for, such number of Ordinary Shares that, together with the number of Ordinary Shares subscribed by the Purchaser at the Initial Closing, will equal 9.9% of the total number of Ordinary Shares outstanding immediately following such issuance (any Ordinary Shares purchased at the Initial Closing and/or the Second Closing, the “**Shares**”), free and clear of all Liens, at a price per share equal to the product of (A) 1.1, multiplied by (B) the VWAP for the twenty (20) Trading Days ending on the date that is five (5) Trading Days prior to such closing (the “**Second Closing**”). The Second Closing shall take place on a date set forth in a notice to be provided by the Purchaser to the Company at the offices of Kirkland & Ellis LLP, 601 Lexington Avenue, New York, NY 10022 (such time and place are designated as the “**Second Closing Date**”).

(c) The term “**Closing**” shall apply to each of the Initial Closing and the Second Closing unless otherwise specified.

**2.2 Delivery and Payment.** At each Closing, subject to the terms and conditions hereof, the Company will instruct the Company’s transfer agent to deliver to the Purchaser, via book entry to the applicable balance account registered in the name of the Purchaser, the number of Shares to be purchased at the Closing by the Purchaser, against payment of the purchase price in U.S. dollars by check or wire transfer made payable to the order of the Company.

### **2.3 Deliveries at each Closing.**

(a) **Deliveries by the Company.** At each Closing, the Company shall deliver or cause to be delivered to the Purchaser the following items (as applicable):

(i) a copy of the notarial deed of incorporation of the Company from a civil law notary dated not more than ten (10) days prior to the applicable Closing Date;

(ii) evidence of (A) the filing of the Listing of Additional Shares notification to NASDAQ as it relates to the Shares to be subscribed for by the Purchaser at such Closing and (B) the registration of the related capital increases with the Dutch Trade Register;

(iii) written confirmation of the book-entry delivery of the Shares to the Purchaser;

(iv) legal opinions of Rutgers Posch Visée Endedijk N.V. and Morgan Lewis & Bockius LLP, the Company’s Dutch and U.S. counsel, each dated as of the applicable Closing Date in form and substance reasonably acceptable to the Purchaser;

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(v) a certificate, dated as of the applicable Closing Date, signed by a managing director of the Company, confirming that the conditions to the applicable Closing set forth in Section 6.1 or Section 6.2, as applicable, have been satisfied; and

(vi) all such other documents, certificates and instruments as the Purchaser may reasonably request in order to give effect to the transactions contemplated hereby and by the other Transaction Documents.

(b) **Deliveries by the Purchaser.** At each Closing, the Purchaser shall deliver or cause to be delivered to the Company the applicable purchase price for the Shares purchased at the applicable Closing, by wire transfer of immediately available funds to one or more accounts



designated by the Company, such designation to be made no later than two (2) Business Days prior to the applicable Closing Date.

**2.4 Withholding.** Purchaser and its agents shall be entitled to deduct and withhold from any consideration payable hereunder any amounts it may be required to deduct and withhold under any applicable Tax Law. Amounts withheld under this Section 2.4 and paid over to the appropriate Governmental Authority shall be treated for all purposes of this Agreement as having been paid to the Person in respect of which such deduction and withholding was made. Purchaser shall provide written notice to the Company, at least three (3) Business Days in advance, as to its intent to impose any such deduction or withholding and the basis therefor.

**3. Representations and Warranties of the Company.** Except as set forth below or in the Company SEC Documents (as defined below) filed prior to the date hereof or in the Draft Annual Report (other than any non-specific forward looking statements set forth in any risk factor section or any non-specific forward-looking or predictive statements), the Company hereby represents and warrants to the Purchaser as of the Signing Date as set forth below.

**3.1 Organization, Good Standing and Qualification.**

(a) The Company is duly incorporated and validly exists as a public limited liability company (*naamloze vennootschap*) under the laws of the Netherlands. The Company has all requisite corporate power and authority to own and operate its properties and assets, to execute and deliver the Transaction Agreements, to issue and sell the Shares, and to carry out the provisions of the Transaction Agreements and to carry on its business as presently conducted and as presently proposed to be conducted. Each of the Company's Subsidiaries (as defined in Section 3.2) is an entity duly incorporated or otherwise organized, validly existing and in good standing (to the extent such concept exists in the relevant jurisdiction) under the Laws of the jurisdiction of its incorporation or organization, as applicable, and has all requisite power and authority to carry on its business to own and use its properties. Neither the Company nor any of its Subsidiaries is in violation or default of any of the provisions of its respective articles of association, charter, certificate of incorporation, bylaws, limited partnership agreement or other organizational or constitutive documents. Each of the Company and its Subsidiaries is duly qualified to do business as a foreign entity and is in good standing (to the extent such concept exists in the relevant jurisdiction) in each jurisdiction in which the conduct of its business or its ownership or leasing of property makes such qualification necessary, except to the extent any

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failure to so qualify has not had and would not reasonably be expected to have a Material Adverse Effect.

(b) During the twelve (12) months preceding the date of this Agreement and of each Closing, neither the Company nor any of its Subsidiaries has taken any action nor, to the Company's Knowledge, have any other steps been taken or Actions commenced or threatened against any of them, for their winding up or dissolution or for any of them to enter into any arrangement, scheme or composition for the benefit of creditors, or for the appointment of a receiver, administrator, liquidator, trustee or similar officer of any of them, or any of their respective properties, revenues or assets.

**3.2 Subsidiaries.** The Company has disclosed all of its subsidiaries required to be disclosed in an exhibit to its Annual Report on Form 20-F (the "**Subsidiaries**"). The Company owns, directly or indirectly, all of the capital stock or other equity interests of each Subsidiary free and clear of any Liens other than pursuant to the Loan Agreement, and all of the issued and outstanding shares of capital stock of each Subsidiary are validly issued and are fully paid and, if applicable in the relevant jurisdiction, non-assessable, and free of preemptive and similar rights to subscribe for or purchase securities.

**3.3 Capitalization.**

(a) The authorized share capital (*maatschappelijk kapitaal*) of the Company, immediately prior to the Signing Date, consists of 60,000,000 Ordinary Shares, 18,429,266 of which are currently issued and outstanding. Under the Company's 2014 Share Incentive Plan, the 2012 Share Incentive Plan and certain ex-plan options (the "Plans"), (i) options to acquire 2,915,268 Ordinary Shares have been granted and are currently outstanding, (ii) 179,068 restricted share units have been granted and are currently outstanding; and (iii) 460,483 Ordinary Shares remain available for future issuance to supervisory or management board members, senior executives, employees and consultants of the Company and its Subsidiaries. In addition, warrants to purchase 170,802 Ordinary Shares are outstanding (the "**Outstanding Warrants**").

(b) Other than the Ordinary Shares reserved for issuance under the Plans and the Outstanding Warrants, and except as may be granted pursuant to the Transaction Agreements, there are no outstanding options, rights (including conversion or preemptive rights and rights of first refusal), proxy or shareholder agreements, or agreements of any kind for the purchase or acquisition from the Company or any of its Subsidiaries of any of its securities, including the Shares to be issued pursuant to the Agreement. No Person is entitled to preemptive rights, rights of first refusal, rights of participation or similar rights with respect to any securities of the Company or any of its Subsidiaries, including with respect to the issuance of Shares contemplated hereby. Except as set forth in the Investor Agreement, there are no voting agreements, registration rights agreements or other agreements of any kind among the Company or any of its Subsidiaries and any other Person relating to the securities of the Company or any of its Subsidiaries, including the Shares to be issued pursuant to this Agreement.

(c) All of the issued and outstanding Ordinary Shares (i) have been duly authorized and validly issued and are fully paid, and (ii) were issued in compliance with all

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applicable Laws concerning the issuance of securities. The Shares have been duly and validly authorized and, when issued and paid for pursuant to this Agreement, (i) will be validly issued, and fully paid, (ii) will form part of the same class of Ordinary Shares and will have the same profit entitlement and voting rights as the Ordinary Shares, (iii) will not be subject to pre-emptive rights, (iv), and shall be free and clear of all Liens, except for restrictions on transfer imposed by the Investor Agreement and applicable securities Laws.

(d) Except as set forth in the Company SEC Documents, (i) neither the Company nor any of its Subsidiaries owns or holds the right to acquire any stock, partnership, interest, joint venture interest or other equity ownership interest in any Person and (ii) the Company owns, directly or indirectly, all of the capital stock or other equity interests of each of its Subsidiaries, free and clear of any Lien.

**3.4 Authorization; Binding Obligations.** Subject to receipt of the AGM General Authorization, all corporate action on the part of the Company, its supervisory and management boards and shareholders necessary for the authorization of the Transaction Agreements, the performance of all

obligations of the Company hereunder and thereunder at each Closing and the authorization, sale, issuance and delivery of the Shares pursuant hereto has or will have been taken on or before the date of each Closing, including the approval by the supervisory board of the Company of the resolution of the management board to issue the Shares and to exclude rights of pre-emption in respect of such issuance, a sufficient amount has been reserved from its authorized share capital to provide for the issuance of the Shares, and no action is required on the part of the Company, its supervisory board, its management board, or its shareholders prior to either Closing for the consummation of the transactions contemplated by the Transaction Agreements. This Agreement has been duly executed and delivered by the Company, and the other Transaction Agreements and instruments referred to herein to which the Company is or is specified to be a party will be duly executed and delivered by the Company as of the Initial Closing. This Agreement constitutes, and the other Transaction Agreements, when executed and delivered, will constitute valid and binding obligations of the Company enforceable in accordance with their terms, except (a) as limited by applicable bankruptcy, insolvency, reorganization, moratorium or other Laws of general application affecting enforcement of creditors' rights, (b) general principles of equity that restrict the availability of equitable remedies and (c) to the extent that the enforceability of the indemnification provisions in the Investor Agreement may be limited by applicable Laws.

### 3.5 Company SEC Documents; Financial Statements; NASDAQ; Indebtedness.

(a) Since February 5, 2014, the Company has timely filed all required reports, schedules, forms, statements and other documents (including exhibits and all other information incorporated therein), and any required amendments to any of the foregoing, with the SEC (the "**Company SEC Documents**"). As of their respective filing dates, each of the Company SEC Documents complied in all material respects with the requirements of the Securities Act and the Exchange Act applicable to such Company SEC Documents, and no Company SEC Documents when filed, declared effective or mailed, as applicable, contained any untrue statement of a material fact or omitted to state a material fact required to be stated therein or necessary in order to make the statements therein, in light of the circumstances under which

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they were made, not misleading. None of the Company's Subsidiaries is subject to the periodic reporting requirements of the Exchange Act. As of the date hereof, there are no outstanding or unresolved comments in comment letters from the SEC staff with respect to any of the Company SEC Documents. To the Company's Knowledge, as of the date hereof, none of the Company SEC Documents is the subject of ongoing SEC review or outstanding investigation.

(b) The financial statements of the Company included in the Company SEC Documents when filed complied as to form in all material respects with applicable accounting requirements and the published rules and regulations of the SEC with respect thereto, have been prepared in accordance with International Financing Reporting Standards ("**IFRS**") as issued by the International Accounting Standards Board applied on a consistent basis during the periods involved (except as may be indicated in the notes thereto) and fairly present in all material respects the financial position of the Company as of the dates thereof and the results of its operations and cash flows for the periods then ended. Except (i) as set forth in the Company SEC Documents or (ii) for liabilities incurred in the ordinary course of business subsequent to the date of the most recent balance sheet contained in the Company SEC Documents, the Company has no liabilities, whether absolute or accrued, contingent or otherwise, other than those that would not, individually or in the aggregate, be material to the Company and its Subsidiaries. Neither the Company nor any of its Subsidiaries has or is subject to any "Off-Balance Sheet Arrangement" (as defined in Item 303(a)(4)(ii) of Regulation S-K promulgated under the Securities Act), except as disclosed in the Company SEC Documents.

(c) The Ordinary Shares are listed on the NASDAQ Global Select Market, and the Company has taken no action designed to, or which would reasonably be expected to have the effect of, terminating the registration of the Ordinary Shares under the Exchange Act or delisting the Ordinary Shares from the NASDAQ Global Select Market. The Company has not received any notification that, and has no Knowledge that, the SEC or NASDAQ is contemplating terminating such listing or registration.

(d) As of the date hereof, other than pursuant to the Loan Agreement or as disclosed in the Company SEC Documents, neither the Company nor any of its Subsidiaries has any outstanding Indebtedness.

**3.6 Obligations to Related Parties.** There are no obligations of the Company or any of its Subsidiaries to supervisory or management board members, senior executives, shareholders, Affiliates, or employees of the Company or any of its Subsidiaries other than (a) for payment of salary for services rendered, (b) reimbursement for reasonable expenses incurred on behalf of the Company and any of its Subsidiaries and (c) for other standard employee benefits made generally available to all employees (including equity award agreements outstanding under any equity incentive plan approved by the supervisory board of the Company). None of the supervisory or management board members, affiliates, senior executives or, to the best of the Company's Knowledge, key employees or shareholders of the Company or any members of their immediate families, is indebted to the Company or any of its Subsidiaries or has any direct or indirect ownership interest in any firm or corporation with which the Company or any of its Subsidiaries is affiliated or with which the Company or any of its Subsidiaries has a business relationship, or any firm or corporation that competes with the Company or any of its Subsidiaries, other than (i) passive investments in publicly traded companies (representing less

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than three percent (3%) of such company) which may compete with the Company or any of its Subsidiaries and (ii) investments by venture capital funds or similar institutional investors with which members of the Company's supervisory board may be affiliated. No supervisory or management board member, senior executive, Affiliate or, to the Knowledge of the Company, shareholder, or any member of their immediate families, is, directly or indirectly, interested in any material contract with the Company or any of its Subsidiaries (other than such contracts as relate to any such person's ownership of share capital or other securities of the Company or any of its Subsidiaries).

**3.7 Compliance with Other Instruments.** Neither the Company nor or any of its Subsidiaries is in violation or default of any term of its articles of association, charter, certificate of incorporation, bylaws, limited partnership agreement, or other organizational or constitutive documents, or of any provision of any mortgage, indenture, contract, lease, agreement, instrument or Contract to which it is party or by which it is bound or of any Order. The execution, delivery, and performance of and compliance with the Transaction Agreements, and the issuance and sale of the Shares pursuant hereto, will not, with or without the passage of time or giving of notice, (i) conflict with or result in a violation of the articles of association, charter, certificate of incorporation, bylaws, limited partnership agreement, or other organizational or constitutive documents of the Company or any of its Subsidiaries, in each case as in effect on the Initial Closing, (ii) result in any violation of any Law or Order to which the Company, any of its Subsidiaries or any of their respective assets is subject, (iii) (A) conflict with or result in a breach, violation of, or constitute a default under, (B) give any third party the right to modify, terminate or

accelerate, or cause any modification, termination or acceleration of, any obligation under, or (C) require Consent under, any material Contract to which the Company or any of its Subsidiaries is a party, or (iv) result in the creation of any Lien upon any of the Company's or any Subsidiary's assets or capital stock, except in the case of any of clauses (ii) and (iv) above, as would not reasonably be expected to have a Material Adverse Effect. Neither the execution, delivery or performance of any Transaction Agreement by the Company, nor the consummation by it of the obligations and transactions contemplated hereby and thereby (including the issuance of the Shares) requires any Consent, other than (i) filings required under applicable U.S. federal and state securities Laws, (ii) the notification of the issuance and sale of the Shares to NASDAQ, (iii) the AGM General Authorization; and (iv) the registration of the related capital increase with the Dutch Trade Register.

**3.8 Litigation.** Except as set forth in the Company SEC Documents filed prior to the date of this Agreement, there is no, and since January 1, 2014 there has not been any, Action pending or, to the Company's Knowledge, threatened, against the Company or any of its Subsidiaries or which the Company intends to initiate which is material. There is no, and since January 1, 2014 there has not been any, Order in effect against the Company or any of its Subsidiaries that is material.

**3.9 Compliance with Laws; Permits.** The Company and its Subsidiaries are not, and since January 1, 2014 have not been, in violation of any applicable Law (including any Health Care Law) in respect of the conduct of its business or the ownership of its properties, which violation would materially and adversely affect the business, assets, liabilities, financial condition, operations or prospects of the Company and its Subsidiaries. No Consents are required to be filed in connection with the execution and delivery of this Agreement or the

issuance of the Shares, except such as have been duly and validly obtained or filed. The Company and each of its Subsidiaries has all franchises, permits, licenses and any similar authority necessary for the conduct of its business as now being conducted by it, the lack of which would reasonably be expected to materially and adversely affect the business, assets, properties or financial condition of the Company and any of its Subsidiaries, and believes it can obtain, without undue burden or expense, any similar authority for the conduct of its business as currently planned to be conducted.

**3.10 Offering Valid.** Assuming the accuracy of the representations and warranties of the Purchaser contained in Section 4.5 hereof, the offer, sale and issuance of the Shares will be exempt from the registration requirements of the Securities Act, and will have been registered or qualified (or are exempt from registration and qualification) under the registration, permit or qualification requirements of all applicable state securities Laws. Neither the Company nor any agent on its behalf has solicited or will solicit any offers to sell or has offered to sell or will offer to sell all or any part of the Shares to any person or persons so as to bring the sale of such Shares by the Company within the registration provisions of the Securities Act or any other securities Laws.

**3.11 Investment Company.** The Company is not, and after giving effect to the transactions contemplated by the Transaction Agreements will not be, an "investment company" or a company "controlled" by an "investment company," within the meaning of the Investment Company Act of 1940, as amended.

**3.12 Sarbanes-Oxley; Internal Controls; Disclosure Controls and Procedures.**

(a) The information set forth under the heading "Item 15: Controls and Procedures" in the Company's most recently filed periodic report under the Exchange Act sets forth an accurate description of the management's evaluation of the Company's internal control over financial reporting (as defined in Rule 13a-15 under the Exchange Act) for the periods described therein.

(b) The Company is in compliance with the applicable requirements of the Sarbanes-Oxley Act of 2002 that are effective as of the date hereof, and the applicable rules and regulations promulgated by the SEC thereunder that are effective as of the date hereof and as of the Initial Closing Date. The Company's certifying officers have evaluated the effectiveness of the Company's disclosure controls and procedures as of the end of the period covered by the Company's most recently filed periodic report under the Exchange Act (such date, the "Evaluation Date"). The Company presented in its most recently filed periodic report under the Exchange Act the conclusions of the certifying officers about the effectiveness of the disclosure controls and procedures based on their evaluations as of the Evaluation Date. Since the Evaluation Date, there have been no changes in the Company's disclosure controls and procedures that have materially affected, or are reasonably likely to materially affect, the effectiveness of the Company's disclosure controls and procedures.

**3.13 Absence of Changes.** Except as set forth in the Company SEC Documents, since December 31, 2014, (i) the Company and its Subsidiaries each has conducted

its business operations in the ordinary course of business consistent with past practice and (ii) there has not occurred any event, change, development, circumstance or condition that, individually or in the aggregate, has had or would be reasonably expected to have a Material Adverse Effect.

**3.14 Tax Matters.** Except as would not reasonably be expected to, individually or in the aggregate, have a Material Adverse Effect:

(a) Except as set forth in the Company SEC Documents, (i) the Company and each of its Subsidiaries has timely prepared and filed all federal and all other material Tax Returns required to have been filed by each of them with all appropriate Governmental Authorities and timely paid all Taxes shown thereon, (ii) all such Tax Returns are true, correct and complete in all respects, (iii) all Taxes that the Company or any of its Subsidiaries is required to withhold or to collect for payment have been duly withheld and collected and paid to the proper Governmental Authority or third party when due, and (iv) to the Company's Knowledge, no claim has ever been made by a Governmental Authority in a jurisdiction where the Company or any of its Subsidiaries does not file Tax Returns that the Company or any of its Subsidiaries is or may be subject to taxation by that jurisdiction;

(b) Except as set forth in the Company SEC Documents, (i) no federal, state, local, or non-U.S. Tax audits or administrative or judicial Tax proceedings are pending or being conducted with respect to the Company or any of its Subsidiaries, (ii) neither the Company nor any of its Subsidiaries has received from any federal, state, local, or non-U.S. taxing authority any (A) written notice indicating an intent to open an audit or other review related to any material Tax, or (B) written notice of deficiency or proposed adjustment for any material amount of Tax proposed, asserted, or assessed by any taxing authority against the Company or any of its Subsidiaries;

(c) Except as set forth in the Company SEC Documents, (i) neither the Company nor any of its Subsidiaries (A) has been a member of an affiliated group filing a consolidated federal income Tax Return (other than a group the common parent of which was the Company) or (B) has any liability for the Taxes of any Person (other than the Company or any of its Subsidiaries) under U.S. Treas. Reg. § 1.1502-6 (or any similar provision of state, local, or non-U.S. Law), as a transferee or successor, by Contract, or otherwise;

(d) Neither the Company nor any of its Subsidiaries has distributed stock of another Person, or has had its stock distributed by another Person, in a transaction that was purported or intended to be governed in whole or in part by Section 355 or 361 of the Code;

(e) Neither the Company nor any of its Subsidiaries is or has been a party to any “listed transaction,” as defined in Section 6707A(c)(2) of the Code and U.S. Treas. Reg. § 1.6011-4(b)(2); and

(f) Neither the Company nor any Subsidiary has ever been, nor will they be at each Closing, a United States Real Property Holding Corporation within the meaning of Code §897(c)(2) during the applicable period specified in Code §897(c)(1)(A)(ii).

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**3.15 Property.** The Company does not own any real property. Except as would not reasonably be expected to, individually or in the aggregate, have a Material Adverse Effect, (i) Company and each of its Subsidiaries has the right to use or occupy the Leased Real Property under valid and binding leases and (ii) the Company and its Subsidiaries have good and valid title to, or a valid license to use or leasehold interest in, all of their respective material tangible assets, free and clear of all Liens (other than Permitted Liens).

### **3.16 Employee Benefits Matters.**

(a) Except as would not reasonably be expected to, individually or in the aggregate, have a Material Adverse Effect: (i) each Employee Benefit Plan (and each related trust, insurance Contract, or fund) has been maintained, funded and administered in accordance with its terms and in compliance with the applicable requirements of Law, including ERISA and the Code and other applicable Laws; and (ii) all contributions, distributions, reimbursements and premium payments due with respect to each Employee Benefit Plan have been timely made or properly accrued. Each Employee Benefit Plan that is intended to meet the requirements of a “qualified plan” under Section 401(a) of the Code has received a favorable determination letter (or may rely on a favorable opinion letter) issued by the United States Internal Revenue Service and to the Company’s Knowledge, nothing has occurred that would reasonably be expected to adversely affect the qualification of such Employee Benefit Plan;

(b) Neither the Company nor any of its Subsidiaries maintains, sponsors, contributes to, has any obligation to contribute to, or has any current or potential liability or obligation under or with respect to (i) a “defined benefit plan” (as such term is defined in Section 3(35) of ERISA), (ii) a “multiple employer plan” (within the meaning of Section 210 of ERISA or Section 413(c) of the Code), or (iii) a “multiemployer plan” as defined in Section 3(37) of ERISA, or (iv) a “multiple employer welfare arrangement” (as such term is defined in Section 3(40) of ERISA); no Employee Benefit Plan provides and neither the Company nor any of its Subsidiaries has any current or potential obligation to provide post-termination or post-retirement health, life or other welfare benefits other than as required under Section 4980B of the Code or any similar state Law; and neither the Company nor any of its Subsidiaries has any current or potential liability or obligation by reason of at any time being treated as a single employer under Section 414 of the Code with any other Person.

(c) To the Company’s Knowledge, (i) there have been no prohibited transactions (as defined in Section 406 of ERISA or Section 4975 of the Code) and no breach of fiduciary duty (as determined under ERISA) with respect to any Employee Benefit Plan, (ii) the Company and its Subsidiaries have, for purposes of each Employee Benefit Plan, correctly classified those individuals performing services for the Company or any of its Subsidiaries as employees or non-employees, and (iii) there do not exist any pending or threatened claims (other than routine undisputed claims for benefits) or Actions with respect to any Employee Benefit Plan.

(d) The transactions contemplated by the Transaction Documents will not (either alone or in combination with another event) cause the acceleration of vesting in, or payment of, any benefits or compensation under any Employee Benefit Plan, will not require the funding of compensation or benefits due to any manager, employee, officer, director, stockholder

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or other service provider (whether current, former or retired) of the Company or any of its Subsidiaries or their beneficiaries and will not otherwise accelerate or increase any liability or obligation under any Employee Benefit Plan..

### **3.17 Labor Matters.**

(a) Neither the Company nor any of its Subsidiaries is a party to or bound by any collective bargaining agreement or other Contract or relationship with any union, labor organization, or other collective bargaining representative. There are no strikes, work stoppages or any other material labor disputes against or affecting the Company or any of its Subsidiaries pending or, to the Company’s Knowledge, threatened, and no such disputes have occurred since January 1, 2014. To the Company’s Knowledge, no union organization or decertification activities are underway or threatened with respect to employees of the Company or any of its Subsidiaries and no such activities have occurred since January 1, 2014.

(b) Except as would not reasonably be expected to, individually or in the aggregate, have a Material Adverse Effect, each of the Company and its Subsidiaries is, and at all times since January 1, 2014, in compliance in all material respects with all applicable Laws respecting employment and employment practices, including provisions thereof relating to terms and conditions of employment, wages and hours, overtime, classification of employees and independent contractors, immigration, and the withholding and payment of social security and other employment Taxes.

(c) Since January 1, 2014, neither the Company nor any of its Subsidiaries has implemented any plant closing or layoff of employees that could implicate the WARN Act.

Effect:

- (a) The Company or its Subsidiaries own all right, title and interest in and to, or have the valid and enforceable right to use pursuant to a written Contract, all Intellectual Property used in the conduct of the business of the Company or any of its Subsidiaries as currently conducted (collectively, the “Company Intellectual Property”) free and clear of any Liens, except Permitted Liens, and to the Company’s Knowledge, the owners of any Intellectual Property licensed to Company or any Subsidiary have taken necessary actions to maintain and protect such Intellectual Property;
- (b) (i) to the Company’s Knowledge, neither the Company nor any of its Subsidiaries has infringed, misappropriated, or otherwise violated, or is currently infringing, misappropriating, or otherwise violating, any Intellectual Property of any other Person, (ii) no Actions are pending or, to the Company’s Knowledge, threatened alleging any of the foregoing, including any unsolicited offers for the Company or any of its Subsidiaries to obtain a license to any Intellectual Property of another Person, and (iii) to the Company’s Knowledge, no Person is infringing, misappropriating or violating the rights of the Company or any of its Subsidiaries with respect to any Company Intellectual Property; and

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- (c) the Company and its Subsidiaries have taken commercially reasonable steps to maintain and protect the secrecy and confidentiality of its and their material Confidential Information. Without limiting the generality of the foregoing, all current and former employees, contractors and consultants of the Company and its Subsidiaries who have participated in the creation of Intellectual Property have executed and delivered to the Company or its Subsidiaries a valid and enforceable agreement providing for the assignment by such Person to the Company or its Subsidiaries of any such Intellectual Property. Company and its Subsidiaries do not have an invention award or compensation system for its employees, contractors, or consultants.

**3.19 Environmental Matters.** Except as would not reasonably be expected to, individually or in the aggregate, have a Material Adverse Effect: (i) no notice, notification, demand, request for information, citation, summons, complaint or Order has been received within the past three years by, and no Action is pending or, to the Company’s Knowledge, threatened by any Person against, the Company or any of its Subsidiaries, and no penalty has been assessed against the Company or any of its Subsidiaries, in each case, with respect to any matters relating to or arising out of any Environmental Law and (ii) the Company and its Subsidiaries are, and since January 1, 2014 have been, in compliance with all applicable Environmental Laws, including any Consent required by Environmental Laws.

**3.20 Brokers and Finders.** Except as set forth in the Company SEC Documents, no Person will have, as a result of the transactions contemplated by the Transaction Documents, any right, interest or claim against or upon the Company or any of its Subsidiaries for any commission, fee or other compensation pursuant to any agreement, arrangement or understanding entered into by or on behalf of the Company or any of its Subsidiaries.

**3.21 Insurances.** Except as set forth in the Company SEC Documents, and except as has not had, and would not reasonably be expected to have, individually or in the aggregate, a Company Material Adverse Effect, (i) all insurance policies (“Policies”) with respect to the business and assets of the Company and its Subsidiaries are in full force and effect, (ii) neither the Company nor any of its Subsidiaries is in breach or default, and neither the Company nor any of its Subsidiaries has taken any action or failed to take any action that, with notice or the lapse of time, would constitute such a breach or default, or permit termination or modification of any of the Policies and (iii) the Company and its Subsidiaries have not received any written notice of cancellation or threatened cancellation of any of the Policies or of any claim pending regarding the Company or any of its Subsidiaries under any of such Policies as to which coverage has been questioned, denied or disputed by the underwriters of such Policies. The Company and its Subsidiaries maintain insurance with reputable insurers in such amounts and against such risks in all material respects as is customary for the industries in which it and its Subsidiaries operate and as the management of the Company has in good faith determined to be prudent and appropriate.

**3.22 Contracts.** Except as set forth in the Company SEC Documents, neither the Company nor any of its Subsidiaries is in violation, default or breach under any of its material Contracts. In this Section 3.22 ‘material’ will mean any Contract entered into with the Company or any of its Subsidiaries that is required to be disclosed as an exhibit to any filing made by the Company pursuant to the Exchange Act.

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**3.23 Application of Takeover Protections.** The Company, the management board and the supervisory board have taken all necessary action, if any, in order to render inapplicable any control share acquisition, business combination, poison pill or other similar anti-takeover provision under the articles of association of the Company, its bye-laws or the Laws of the Netherlands that is or could become applicable to, or is or could be to the detriment of, the Purchaser as a result of the Purchaser and the Company fulfilling their respective obligations or exercising their respective rights under the Transaction Documents, including as a result of the issuance or ownership of the Shares and ordinary shares of the Company issuable upon exercise of the Warrant Agreements.

**3.24 Anti-Corruption and Anti-Bribery Laws.** Neither the Company and its Subsidiaries, nor, to the Knowledge of the Company, any of their respective officers, directors, employees, agents, representatives, consultants, or other persons associated with or acting for or on behalf of the Company or its Subsidiaries, has, directly or indirectly, in connection with the operation of their business: (a) made, offered or promised to make or offer any payment, loan or transfer of anything of value, including any reward, advantage or benefit of any kind, to or for the benefit of any government official, candidate for public office, political party or political campaign, for the purpose of (A) influencing any act or decision of such government official, candidate, party or campaign, (B) inducing such government official, candidate, party or campaign to do or omit to do any act in violation of a lawful duty, (C) obtaining or retaining business for or with any person, (D) expediting or securing the performance of official acts of a routine nature, or (E) otherwise securing any improper advantage, in each case, in violation of any applicable anticorruption or anti-bribery Law; (b) paid, offered or promised to pay or offer any bribe, payoff, influence payment, kickback, unlawful rebate, or other similar unlawful payment of any nature; (c) made, offered or promised to make or offer any unlawful contributions, gifts, entertainment or other unlawful expenditures; (d) established or maintained any unlawful fund of corporate monies or other properties; (e) created or caused the creation of any false or inaccurate books and records of Parent related to any of the foregoing; or (f) otherwise violated any provision of the Foreign Corrupt Practices Act of 1977, 15 U.S.C. §§ 78dd-1, et seq., or any other applicable anti-corruption or anti-bribery Law. For purposes of this provision, “government official” includes any officer or employee of a government or any department, agency or instrumentality thereof (including wholly or partially owned enterprises or institutions), or of a public international organization, or any person acting in an official capacity for or on behalf of any such government or department, agency or instrumentality, or for or on behalf of any such public international organization.

**3.25 Economic Sanctions.** None of the Company or its Subsidiaries or, to the Knowledge of the Company, their respective directors, officers, employees or agents (i) is a person with whom transactions are prohibited or limited under any applicable economic sanctions Laws; or (ii) within the last five (5) years has done business in or with Cuba, Iran, Sudan, North Korea or Syria, or any Person that is the target of sanctions by the United States or The Netherlands. Within the past five years, none of the Company or its Subsidiaries has made any voluntary disclosures to applicable Governmental Authorities under applicable economic sanctions Laws or applicable export control Laws and, to the Knowledge of the Company, none of the Company or its Subsidiaries has been the subject of any governmental investigation or inquiry regarding the compliance of the Company or its Subsidiaries with such Laws, nor have

the Company or its Subsidiaries been assessed any fine or penalty in regard to compliance with such Laws.

**4. Representations and Warranties of the Purchaser.** The Purchaser hereby represents and warrants to the Company as follows as of the Signing Date:

**4.1 Organization; Good Standing.** The Purchaser is a corporation duly organized, validly existing and in good standing under the laws of the State of Delaware. The Purchaser has or will have all requisite power and authority to enter into the Transaction Agreements, to subscribe for the Shares and to perform its obligations under and to carry out the other transactions contemplated by the Transaction Agreements.

**4.2 Requisite Power and Authority.** The Purchaser has all necessary power and authority to execute and deliver the Transaction Agreements and to carry out their provisions. All action on the Purchaser's part required for the lawful execution and delivery of the Transaction Agreements has been taken. Upon their execution and delivery, the Transaction Agreements will be valid and binding obligations of the Purchaser, enforceable in accordance with their terms, except (a) as limited by applicable bankruptcy, insolvency, reorganization, moratorium or other Laws of general application affecting enforcement of creditors' rights, (b) as limited by general principles of equity that restrict the availability of equitable remedies and (c) to the extent that the enforceability of the indemnification provisions of the Investor Agreement may be limited by applicable Laws.

**4.3 No Conflicts.** The execution, delivery and performance of the Transaction Agreements and compliance with the provisions thereof by the Purchaser do not and shall not: (a) violate any provision of applicable Law or any ruling, writ, injunction, order, permit, judgment or decree of any Governmental Authority, (b) constitute a breach of, or default under (or an event which, with notice or lapse of time or both, would become a default under) or conflict with, or give rise to any right of termination, cancellation or acceleration of, any agreement, arrangement or instrument, whether written or oral, by which the Purchaser or any of its assets, are bound, or (c) violate or conflict with any of the provisions of the Purchaser's certificate of incorporation or bylaws, except as would not impair or adversely affect the ability of the Purchaser to consummate the transactions contemplated pursuant to the Transaction Agreements and perform its obligations under the Transaction Agreements and except, in the case of subsections (a) and (b) as would not have a material adverse effect on the Purchaser.

**4.4 No Governmental Authority or Third Party Consents.** No Consent is required to be obtained by the Purchaser in connection with the authorization, execution and delivery of any of the Transaction Agreements or with the subscription for the Shares.

**4.5 Investment Representations.** Purchaser understands that the Shares have not been registered under the Securities Act. The Purchaser also understands that the Shares are being offered and sold pursuant to an exemption from registration contained in the Securities Act based in part upon the Purchaser's representations contained in the Agreement. The Purchaser hereby represents and warrants as follows:

**(a) Purchaser Bears Economic Risk.** The Purchaser has substantial experience in evaluating and investing in private placement transactions of securities in companies similar to the Company and is capable of evaluating the merits and risks of its investment in the Company and has the capacity to protect its own interests. The Purchaser must bear the economic risk of this investment indefinitely unless the Shares are registered pursuant to the Securities Act, or an exemption from registration is available. The Purchaser understands that the Company has no present intention of registering the Shares. The Purchaser also understands that there is no assurance that any exemption from registration under the Securities Act will be available and that, even if available, such exemption may not allow the Purchaser to transfer all or any portion of the Shares under the circumstances, in the amounts or at the times the Purchaser might propose.

**(b) Acquisition for Own Account.** The Purchaser is acquiring the Shares for the Purchaser's own account for investment only, and not with a view towards their distribution.

**(c) Accredited Investor.** The Purchaser represents that it is an accredited investor within the meaning of Regulation D under the Securities Act.

**(d) Company Information.** The Purchaser has had an opportunity to discuss the Company's business, management and financial affairs with management of the Company and has had the opportunity to review the Company's operations and facilities. The Purchaser has also had the opportunity to ask questions of, and receive answers from, the Company and its management regarding the terms and conditions of this investment. The foregoing does not affect the Company's representations and warranties contained in Section 3.

**4.6 Transfer Restrictions.** The Purchaser understands that the Shares shall be subject to restrictions on resale pursuant to applicable securities Laws and that the applicable balance account of the Purchaser with the Company's transfer agent shall bear a restriction with the effect of the following legends:

**(a)** "These securities have not been registered under the Securities Act of 1933. They may not be sold, offered for sale, pledged or hypothecated in the absence of a registration statement in effect with respect to the securities under the Securities Act or an opinion of counsel (which counsel shall be reasonably satisfactory to uniQure N.V.) that such registration is not required or unless sold pursuant to Rule 144 of the Securities Act.";

(b) any legend required by other applicable securities Laws; and

(c) “The securities represented by this certificate are subject to and shall be transferable only upon the terms and conditions of an Investor Agreement dated as of April 6, 2015, by and between uniQure N.V. and Bristol-Myers Squibb Company, a copy of which is on file with the Secretary of uniQure N.V.”

**4.7 Financial Assurances.** As of the date hereof and as of each Closing, the Purchaser has and will have access to funds in an amount sufficient to pay to the Company the aggregate purchase price at each Closing.

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## **5. Covenants and Agreements.**

**5.1 Further Assurances.** Subject to the terms and conditions of this Agreement, each of the Company and the Purchaser agrees to use its reasonable best efforts to take, or cause to be taken, all actions, and to do, or cause to be done, and assist the other party hereto in doing, all things reasonably necessary, proper or advisable to obtain satisfaction of the conditions precedent to the consummation of the transactions contemplated at the Initial Closing and the Second Closing, including: (a) obtaining all necessary Consents and the making of all filings and the taking of all steps as may be necessary to obtain Consent from, or to avoid an Action by, any Governmental Authority, (b) the defending of any Actions challenging this Agreement or any other Transaction Agreements or the consummation of the transactions contemplated hereby or thereby, including seeking to have any stay or temporary restraining order entered by any court or other Governmental Authority vacated or reversed and (c) the execution and delivery of any additional instruments necessary to consummate the transactions contemplated by, and to fully carry out the purposes of, this Agreement and the other Transaction Agreements.

### **5.2 Annual General Meeting.**

(a) The Company agrees to use its best efforts to, at the next regularly scheduled annual general meeting of the Company which annual meeting shall in no event occur later than June 30, 2015 (the “**2015 Annual General Meeting**”), obtain each of the AGM General Authorization and the AGM BMS Transaction Authorization, and as promptly as reasonably practicable in accordance with such timing (and with due observance of the term for convening the meeting as set forth in article 8.3.1 of its Charter), the Company will prepare a convocation notice to be sent to the Company’s shareholders in connection with the 2015 Annual General Meeting (the “**Convocation Notice**”).

(b) The Company shall (i) timely circulate the Convocation Notice in accordance with its Charter and applicable Law; (ii) present to the 2015 Annual General Meeting proposals to approve each of the AGM General Authorization and the AGM BMS Transaction Authorization; (iii) assure that such proposals shall be validly put on the agenda of the 2015 Annual General Meeting; and (iv) assure that the Convocation Notice complies in all material respects with the provisions of all applicable Law, its articles of association and other organizational or constitutive documents, and the NASDAQ listing rules. The Convocation Notice shall include such information relating to the transactions contemplated by the Transaction Agreements that is sufficient for the shareholders of the Company to provide the AGM BMS Transaction Authorization. The Company shall provide the Purchaser and its representatives with a reasonable opportunity to review and comment on the Convocation Notice prior to its being circulated to the shareholders of the Company, and the Company shall consider in good faith any comments proposed by the Purchaser and its representatives for inclusion therein.

(c) The Convocation Notice shall include the Company’s unanimous recommendation that the shareholders vote in favor of each of the AGM General Authorization and the AGM BMS Transaction Authorization. The Company will cause the Convocation Notice, at the time of the mailing of the Convocation Notice or any amendments or supplements

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thereto, and at the time of the 2015 Annual General Meeting, to not contain any untrue statement of a material fact or omit to state any material fact required to be stated therein or necessary in order to make the statements therein, in light of the circumstances under which they were made, not misleading.

(d) If neither the AGM BMS Transaction Authorization nor the AGM General Authorization is obtained at the 2015 Annual General Meeting, then the Company shall use its best efforts to obtain the AGM General Authorization as soon as practicable thereafter. Until the AGM General Authorization has been obtained, the Company shall use its best efforts to solicit from its shareholders proxies in favor of the AGM General Authorization and to obtain the AGM General Authorization at each (annual or extraordinary) general meeting of the Company occurring after the 2015 Annual General Meeting.

**5.3 No Restrictions on Transfer.** Subject to restrictions on transfer imposed by the Transaction Agreements and applicable securities Laws, the Shares shall be freely transferrable.

**5.4 Securities Law Disclosure; Publicity.** No public release or announcement concerning the transactions contemplated hereby or by any other Transaction Agreement shall be issued by the Company or the Purchaser without the prior consent of the Company (in the case of a release or announcement by the Purchaser) or the Purchaser (in the case of a release or announcement by the Company) (which consents shall not be unreasonably withheld, conditioned or delayed), except for any such release or announcement as may be required by Law or the applicable rules or regulations of any securities exchange or securities market, in which case the Company or the Purchaser, as the case may be, shall allow the Purchaser or the Company, as applicable, to the extent reasonably practicable in the circumstances, reasonable time to comment on such release or announcement in advance of such issuance. The provisions of this Section 5.4 shall not restrict the ability of the Company to summarize or describe the transactions contemplated by this Agreement in any prospectus or similar offering document or any other filing that is made pursuant to the terms of the Exchange Act so long as the Purchaser is provided a reasonable opportunity to review and comment on such disclosure in advance of the filing or other public dissemination of any such document.

**5.5 NASDAQ Matters.** Prior to each Closing, the Company shall (i) take all actions which are necessary, including providing appropriate notice to NASDAQ of the transactions contemplated by this Agreement, for the Shares purchased at such Closing to remain listed on the NASDAQ Global Select Market and (ii) comply with all listing, reporting, filing, and other obligations under the rules of NASDAQ and of the SEC.

**5.6 Interim Operations of the Company.** Prior to each Closing Date or the earlier termination of this Agreement in accordance with its terms, the Company shall not voluntarily delist from the NASDAQ Global Select Market. Between the date hereof and the Second Closing Date, the Company will not amend its articles of association in a manner that is materially adverse to the Purchaser's rights under the Transaction Agreements, and will not take or omit to take any action, or permit its Subsidiaries to take or to omit to take any action, that would or could reasonably be expected to have a Material Adverse Effect.

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**5.7 Integration.** The Company shall not sell, offer for sale or solicit offers to buy or otherwise negotiate in respect of any security (as defined in the Securities Act) that would be integrated with the offer or sale of the Shares to be issued to the Purchaser hereunder for purposes of the rules and regulations of any of the following markets or exchanges on which the Common Stock or the Company is listed or quoted for trading on the date in question: the Pink OTC Markets, the OTC Bulletin Board, the American Stock Exchange, the NASDAQ Capital Market, the NASDAQ Global Market, the NASDAQ Global Select Market or the New York Stock Exchange.

**5.8 Notification.** After the date hereof and prior to the Second Closing Date, the Company shall promptly deliver to the Purchaser a written notice of any event or development that would, or could reasonably be expected to, result in any condition to Closing set forth in Section 6.1 or Section 6.2, as applicable, not to be satisfied.

**5.9 Use of Proceeds.** The net proceeds received by the Company from each Closing shall be used for general corporate purposes at the direction of the supervisory board of the Company.

## **6. Conditions to Closing.**

**6.1 Conditions to Purchaser's Obligations at the Initial Closing.** The Purchaser's obligation to purchase Shares at the Initial Closing is subject to the satisfaction, at or prior to the Initial Closing Date, of the following conditions (unless waived in writing by the Purchaser):

(a) **Representations and Warranties.** The representations and warranties made by the Company in Section 3 hereof shall be true and correct as of the date hereof and as of the Initial Closing Date as if made on such date, except to the extent such representations and warranties are specifically made as of a particular date, in which case such representations and warranties shall be true and correct as of such date.

(b) **Performance of Obligations.** The Company shall have performed and complied with all agreements and conditions herein required to be performed or complied with by the Company on or before the Initial Closing Date.

(c) **Legal Investment.** The sale and issuance of the Shares shall be legally permitted by all Laws to which the Purchaser and the Company are subject.

(d) **No Orders.** No Order shall be in effect preventing the consummation of the transactions contemplated by the Transaction Agreements.

(e) **Closing Deliverables.** The Company shall deliver or cause to be delivered to the Purchaser all items (as applicable), listed in Section 2.3(a).

(f) **Collaboration Agreement.** The Company shall have executed the Collaboration Agreement, the Effective Date of the Collaboration Agreement shall have occurred, no material breach by the Company of any term of or obligation under the

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Collaboration Agreement shall have occurred and be continuing, and the Collaboration Agreement shall not have been terminated in accordance with its terms.

(g) **Consents, Permits, and Waivers.** All Consents necessary or appropriate for consummation of the transactions contemplated by the Transaction Agreements shall have been obtained, including either the AGM BMS Transaction Authorization or the AGM General Authorization and the approval of the supervisory board of the Company. All filings to be made under the HSR Act or any other similar antitrust Laws (including but not limited to the Netherlands), with respect to the Transaction Agreement and the transactions contemplated hereby and thereby, shall have been made and the applicable waiting period, including all extensions thereof, under the HSR Act or any other similar antitrust Laws (including but not limited to the Netherlands), shall have expired or been terminated.

(h) **MAE.** No Material Adverse Effect shall have occurred and be continuing.

(i) **The Company's NASDAQ Listing.** The Company's Ordinary Shares shall continue to be listed on the NASDAQ Global Select Market.

(j) **Annual Report.** The Company's Annual Report on Form 20-F in respect of the year ended December 31, 2014 shall have been filed with the SEC in substantially the same form as the Draft Annual Report, together with a signed opinion from the Company's outside independent auditor that does not contain any "going concern" or other qualifications.

**6.2 Conditions to Purchaser's Obligations at the Second Closing.** The Purchaser's obligation to subscribe for the Purchaser's Shares at the Second Closing shall be subject to the satisfaction, at or prior to the Second Closing Date, of the following conditions (unless waived in writing by the Purchaser):

(a) The Initial Closing shall have occurred.



(b) **Representations and Warranties.** The representations and warranties made by the Company in Section 3 hereof shall be true and correct on the date hereof and the Second Closing Date as if made on the Second Closing Date, except to the extent such representations and warranties are specifically made as of a particular date, in which case such representations and warranties shall be true and correct as of such date.

(c) **Performance of Obligations.** The Company shall have performed and complied with all agreements and conditions herein required to be performed or complied with by the Company on or before the Second Closing Date.

(d) **Legal Investment.** The sale and issuance of the Shares shall be legally permitted by all Laws to which the Purchaser and the Company are subject.

(e) **No Injunction or Restraints.** No Order shall be in effect preventing the consummation of the transactions contemplated by the Transaction Agreements.

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(f) **Closing Deliverables.** The Company shall deliver or cause to be delivered to the Purchaser all items (as applicable), listed in Section 2.3(a).

(g) **Collaboration Agreement.** No material breach by the Company of any terms of or obligations under the Collaboration Agreement shall have occurred and be continuing, and the Collaboration Agreement shall not have been terminated in accordance with its terms.

(h) **Consents, Permits, and Waivers.** All Consents necessary or appropriate for consummation of the transactions contemplated by the Transaction Agreements shall have been obtained.

**6.3 Conditions to Company's Obligations at the Initial Closing.** The Company's obligation to issue and sell Shares at the Initial Closing is subject to the satisfaction, on or prior to the Initial Closing Date, of the following conditions (unless waived in writing by the Company):

(a) **Representations and Warranties.** The representations and warranties in Section 4 made by the Purchaser shall be true and correct in all material respects as of the Initial Closing Date.

(b) **Performance of Obligations.** The Purchaser shall have performed and complied with all agreements and conditions herein required to be performed or complied with by the Purchaser on or before the Initial Closing Date.

(c) **Legal Investment.** The sale and issuance of the Shares shall be legally permitted by all Laws to which the Purchaser and the Company are subject.

(d) **No Order.** No Order shall be in effect preventing the consummation of the transactions contemplated by the Transaction Agreements.

(e) **Closing Deliverables.** The Purchaser shall deliver or cause to be delivered to the Company all items (as applicable), listed in Section 2.3(b).

(f) **Collaboration Agreement.** The Collaboration Agreement shall not have been terminated in accordance with its terms.

(g) **Consents, Permits, and Waivers.** All Consents necessary or appropriate for consummation of the transactions contemplated by the Transaction Agreements shall have been obtained. All filings to be made under the HSR Act or any other similar antitrust Laws (including but not limited to the Netherlands), with respect to the Transaction Agreement and the transactions contemplated hereby and thereby, shall have been made and the applicable waiting period, including all extensions thereof, under the HSR Act or any other similar antitrust Laws (including but not limited to the Netherlands), shall have expired or been terminated.

**6.4 Conditions to Company's obligations of the Second Closing.** The Company's obligation to issue and sell Shares at the Second Closing is subject to the satisfaction,

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on or prior to the Second Closing Date, of the following conditions (unless waived in writing by the Company):

(a) **Representations and Warranties.** The representations and warranties made by the Purchaser in Section 4 hereof shall be true and correct as of on the Second Closing Date, except to the extent such representations and warranties are specifically made as of a particular date, in which case such representations and warranties shall be true and correct as of such date.

(b) **Performance of Obligations.** The Purchaser shall have performed and complied with all agreements and conditions herein required to be performed or complied with by the Purchaser on or before the Second Closing Date.

(c) **Legal Investment.** On the Second Closing Date, the sale and issuance of the Shares shall be legally permitted by all Laws to which the Purchaser and the Company are subject.

(d) **No Injunction or Restraints.** No Order shall be in effect preventing the consummation of the transactions contemplated by the Transaction Agreements.

(e) **Closing Deliverables.** The Purchaser shall deliver or cause to be delivered to the Company all items (as applicable), listed in Section 2.3(b).

(f) **Consents, Permits, and Waivers.** All Consents necessary or appropriate for consummation of the transactions contemplated by the Transaction Agreements shall have been obtained.

## 7. Miscellaneous.

7.1 **Termination.** This Agreement and the obligations of the parties hereunder may be terminated by:

- (a) the Company and the Purchaser, by providing mutual written consent to terminate;
- (b) either the Company or the Purchaser, upon written notice to the other no earlier than three (3) Trading Days after [\*\*] (the “**Original Termination Date**”), if the Original Termination Date cannot be or has not been validly extended pursuant to this Section 7.1(b), and if the Initial Closing shall not have been consummated by the Original Termination Date; provided, however, that the Original Termination Date may be extended to [\*\*] (the “**Final Termination Date**”) by either the Company or the Purchaser, in their sole discretion, upon written notice to the other on or within five (5) Trading Days prior to the Original Termination Date, if the Initial Closing Date shall not have been consummated by the Original Termination Date solely as the result of a failure to satisfy the condition set forth in Sections 6.1(g) and 6.3(g) as of the Original Termination Date; provided further, however, that the right to terminate this Agreement under this Section 7.1(b) shall not be available to any party whose failure to fulfill any obligation under this Agreement has been the cause of, or resulted in, the failure to

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consummate the Initial Closing prior to the Original Termination Date or the Final Termination Date, as applicable;

- (c) either the Company or the Purchaser, upon written notice to the other no earlier than three (3) Trading Days after December 31, 2015 if the Second Closing Date shall not have occurred by such date; provided, however, that the right to terminate this Agreement under this Section 7.1(c) shall not be available to any party whose failure to fulfill any obligation under this Agreement has been the cause of, or resulted in, the failure to consummate the Second Closing prior to December 31, 2015;

- (d) the Company, upon written notice to the Purchaser, so long as the Company is not then in material breach of its representations, warranties, covenants or agreements under this Agreement such that any of the conditions set forth in Section 6.1 and Section 6.2, as applicable, would not be satisfied by Second Closing Date, (i) upon a material breach of any covenant or agreement on the part of the Purchaser set forth in this Agreement, or (ii) if any representation or warranty of the Purchaser shall have been or become untrue, in each case such that any of the conditions set forth in Section 6.2(b) would not be satisfied by the Second Closing Date, and such breach has not been waived by the Company or cured by the Purchaser, as applicable, within fifteen (15) days after receipt by the Purchaser of written notice thereof by the Company or is not reasonably capable of being cured by the Purchaser prior to the Original Termination Date or the Final Termination Date, as applicable

- (e) the Purchaser, upon written notice to the Company, so long as the Purchaser is not then in breach of its representations, warranties, covenants or agreements under this Agreement such that any of the conditions set forth in Section 6.2(b), would not be satisfied by Second Closing Date, upon a breach of any covenant or agreement on the part of the Company set forth in this Agreement, or if any representation or warranty of the Company shall have been or become untrue, in each case such that any of the conditions set forth in Section 6.1 and Section 6.2 would not be satisfied by the Second Closing Date, and such breach has not been waived by the Purchaser or cured by the Company, as applicable, within 15 days after receipt by the Company of written notice thereof by the Purchaser or is not reasonably capable of being cured by the Company prior to the Original Termination Date or the Final Termination Date, as applicable; and

- (f) by the Company or Purchaser, upon notice to the other, if there shall be any Law that makes consummation of the transactions contemplated by this Agreement illegal or otherwise prohibited, or a Governmental Authority of competent jurisdiction has issued an Order permanently enjoining or otherwise prohibiting or restraining the consummation of the transactions contemplated by this Agreement, and such Order has become final and non-appealable; provided, however, that the right to terminate this Agreement pursuant to this Section 7.1(f) shall not be available to any party whose breach of any provision of this Agreement results in or causes such Order or who is not in compliance with its obligations under Section 5.1.

In the event of termination of this Agreement pursuant to Section 7.1 by either Purchaser or the Company, this Agreement will become void and have no further force or effect, without any liability or obligation of the Purchaser, other than as set forth in Sections 5.4 and Article VII,

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which will each survive any termination of this Agreement; provided, however, that nothing herein will relieve the Purchaser from any liability for any actual fraud of the Purchaser occurring prior to such termination.

7.2 **Governing Law; Waiver of Jury Trial.** This Agreement shall be governed by and construed in accordance with the Laws of the State of New York, without regard to the conflict of laws principles thereof that would require the application of the Law of any other jurisdiction. The parties irrevocably and unconditionally submit to the exclusive jurisdiction of the United States District Court for the Southern District of New York solely and specifically for the purposes of any action or proceeding arising out of or in connection with this Agreement. EACH OF THE PARTIES TO THIS AGREEMENT HEREBY AGREES THAT JURISDICTION AND VENUE IN ANY SUIT, ACTION OR PROCEEDING BROUGHT BY ANY PARTY ARISING OUT OF OR RELATING TO THIS AGREEMENT (INCLUDING ANY SUIT, ACTION OR PROCEEDING SEEKING EQUITABLE RELIEF) SHALL PROPERLY AND EXCLUSIVELY LIE IN THE STATE AND FEDERAL COURTS LOCATED IN THE STATE OF NEW YORK (THE “**CHOSEN COURTS**”). EACH PARTY HERETO FURTHER AGREES NOT TO BRING ANY SUCH SUIT, ACTION OR PROCEEDING IN ANY COURT OTHER THAN THE CHOSEN COURTS PURSUANT TO THE FOREGOING SENTENCE (OTHER THAN UPON APPEAL). BY EXECUTION AND DELIVERY OF THIS AGREEMENT, EACH PARTY IRREVOCABLY SUBMITS TO THE JURISDICTION OF THE CHOSEN COURTS FOR ITSELF AND IN RESPECT OF ITS PROPERTY WITH RESPECT TO SUCH SUIT, ACTION OR PROCEEDING. THE PARTIES HERETO IRREVOCABLY AGREE THAT VENUE WOULD BE PROPER IN EACH OF THE CHOSEN COURTS, AND HEREBY WAIVE ANY OBJECTION THAT ANY SUCH CHOSEN COURT IS AN IMPROPER OR INCONVENIENT FORUM FOR THE RESOLUTION OF SUCH SUIT, ACTION OR PROCEEDING. TO THE EXTENT NOT PROHIBITED BY APPLICABLE LAW WHICH CANNOT BE WAIVED, EACH PARTY HERETO HEREBY

WAIVES AND COVENANTS THAT IT WILL NOT ASSERT (WHETHER AS PLAINTIFF, DEFENDANT OR OTHERWISE) ANY RIGHT TO TRIAL BY JURY IN ANY FORUM IN RESPECT OF ANY ISSUE OR ACTION, CLAIM, CAUSE OF ACTION OR SUIT (IN CONTRACT, TORT OR OTHERWISE) INQUIRY, PROCEEDING OR INVESTIGATION ARISING OUT OF OR BASED UPON THIS AGREEMENT OR THE SUBJECT MATTER HEREOF OR IN ANY WAY CONNECTED WITH OR RELATED OR INCIDENTAL TO THE TRANSACTIONS CONTEMPLATED HEREBY, IN EACH CASE WHETHER NOW EXISTING OR HEREAFTER ARISING. EACH PARTY HERETO ACKNOWLEDGES THAT IT HAS BEEN INFORMED BY THE OTHER PARTIES HERETO THAT THIS SECTION 7.2 CONSTITUTES A MATERIAL INDUCEMENT UPON WHICH THEY ARE RELYING AND WILL RELY IN ENTERING INTO THIS AGREEMENT. ANY PARTY HERETO MAY FILE AN ORIGINAL COUNTERPART OR A COPY OF THIS SECTION 7.2 WITH ANY COURT AS WRITTEN EVIDENCE OF THE CONSENT OF EACH SUCH PARTY TO THE WAIVER OF ITS RIGHT TO TRIAL BY JURY.

**7.3 Survival.** The representations, warranties, covenants and agreements made herein shall survive for a period of one (1) year following the Second Closing Date. All statements as to factual matters contained in any certificate or other instrument delivered by or on behalf of the Company pursuant hereto in connection with the transactions contemplated hereby shall be deemed to be representations and warranties by the Company hereunder solely as

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of the date of such certificate or instrument and shall survive in accordance with the immediately preceding sentence. The representations, warranties, covenants and obligations of the Company, and the rights and remedies that may be exercised by the Purchaser, shall not be limited or otherwise affected by or as a result of any information furnished to, or any investigation made by or knowledge of, the Purchaser or its representatives.

**7.4 Successors and Assigns.** Except as otherwise expressly provided herein, the provisions hereof shall inure to the benefit of, and be binding upon the parties hereto and their respective successors, assigns, heirs, executors and administrators and shall inure to the benefit of and be enforceable by each person who shall be a holder of the Shares from time to time; *provided, however*, that prior to the receipt by the Company of adequate written notice of the transfer of any Shares specifying the full name and address of the transferee, the Company may deem and treat the person listed as the holder of such Shares in its records as the absolute owner and holder of such Shares for all purposes. This Agreement may not be assigned by any party hereto without the consent of the other party, provided, that the Purchaser may assign its rights and obligations hereunder in whole or in part to any Affiliate of the Purchaser, provided that in the case of such assignment the Purchaser shall not be relieved of its obligations hereunder, or to any transferee to whom Shares are properly transferred after a particular Closing pursuant to the terms of the Transaction Agreements.

**7.5 Entire Agreement.** This Agreement, the exhibits and schedules hereto, the other Transaction Agreements, the Collaboration Agreement and the other documents delivered pursuant hereto constitute the full and entire understanding and agreement between the parties with regard to the subjects hereof and no party shall be liable for or bound to any other in any manner by any oral or written representations, warranties, covenants and agreements except as specifically set forth herein and therein.

**7.6 Severability.** In the event one or more of the provisions of this Agreement should, for any reason, be held to be invalid, illegal or unenforceable in any respect, such invalidity, illegality or unenforceability shall not affect any other provisions of this Agreement, and this Agreement shall be construed as if such invalid, illegal or unenforceable provision had never been contained herein. Upon such determination that any provision of this Agreement, or the application of any such provision, is invalid, illegal, void or unenforceable, the Company and the Purchaser shall negotiate in good faith to modify this Agreement so as to effect the original intent of the Company and the Purchaser as closely as possible to the fullest extent permitted by Law in an acceptable manner to the end that the transactions contemplated hereby and the other Transaction Agreements are fulfilled to the greatest extent possible.

**7.7 Amendment.** No provision in this Agreement shall be supplemented, deleted or amended except in a writing executed by an authorized representative of each of the Purchaser and the Company. Any amendment effected in accordance with this Section 7.7 shall be binding upon each holder of Shares purchased under this Agreement at the time outstanding, each future holder of all such Shares, and the Company, and any amendment not effected in accordance with this Section 7.7 shall be void and of no effect.

**7.8 Waivers; Delays or Omissions.** It is agreed that no delay or omission to exercise any right, power or remedy accruing to any party, upon any breach, default or

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noncompliance by another party under this Agreement or the Investor Agreement, shall impair any such right, power or remedy, nor shall it be construed to be a waiver of any such breach, default or noncompliance, or any acquiescence therein, or of or in any similar breach, default or noncompliance thereafter occurring. It is further agreed that any Consent of any kind or character on any party's part of any breach, default or noncompliance under this Agreement or the Investor Agreement or any waiver on such party's part of any provisions or conditions of the Agreement or the Investor Agreement must be in writing and shall be effective only to the extent specifically set forth in such writing. All remedies, either under this Agreement, the Investor Agreement, by Law, or otherwise afforded to any party, shall be cumulative and not alternative. Any waiver effected in accordance with this Section 7.8 shall be binding upon each holder of Shares purchased under this Agreement at the time outstanding, each future holder of all such Shares, and the Company, and any waiver not effected in accordance with this Section 7.8 shall be void and of no effect.

**7.9 Equitable Relief.** Each of the Company and the Purchaser hereby acknowledges and agrees that the failure of the Company to perform its respective agreements and covenants hereunder will cause irreparable injury to the Purchaser, for which damages, even if available, will not be an adequate remedy. Accordingly, the Company hereby agrees that the Purchaser shall be entitled to the issuance of equitable relief by any court of competent jurisdiction to compel performance of the Company's obligations.

**7.10 Waiver of Conflicts.** Each party to this Agreement acknowledges that Morgan, Lewis & Bockius LLP ("**MLB**"), outside counsel to the Company, has in the past performed and is or may now or in the future represent the Purchaser or its Affiliates in matters unrelated to the transactions contemplated by this Agreement (the "**Financing**"), including representation of the Purchaser or its Affiliates in matters of a similar nature to the Financing. The applicable rules of professional conduct require that MLB inform the parties hereunder of this representation and obtain their consent. MLB has served as outside counsel to the Company and has negotiated the terms of the Financing solely on behalf of the Company. The Company and the Purchaser hereby (a) acknowledge that they have had an opportunity to ask for and have obtained information relevant to such representation, including disclosure of the reasonably foreseeable adverse consequences of such representation; (b) acknowledge that with respect to the Financing, MLB has represented solely the

Company, and not the Purchaser or any shareholder, director or employee of the Company or the Purchaser; and (c) gives its informed consent to MLB's representation of the Company in the Financing.

**7.11 Notices.** All notices required or permitted hereunder shall be in writing and shall be deemed effectively given: (a) upon personal delivery to the party to be notified, (b) when sent by confirmed electronic mail, telex or facsimile if sent during normal business hours of the recipient, if not, then on the next Trading Day, (c) five (5) days after having been sent by registered or certified mail, return receipt requested, postage prepaid or (d) one (1) day after deposit with a nationally recognized overnight courier, specifying next day delivery, with written verification of receipt. All communications shall be sent:

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For the Company:

uniQure N.V.  
Meibergdreef 61  
Amsterdam 1105 BA  
The Netherlands  
Attention: Jörn Aldag, Chief Executive Officer  
Email:

With a copy to:

Morgan, Lewis & Bockius LLP  
Condor House, 5-10 St. Paul's Churchyard  
London, EC4M 8AL  
United Kingdom  
Attention: Timothy J. Corbett  
Email: tcorbett@morganlewis.com

For the Purchaser:

Bristol-Myers Squibb Company  
345 Park Avenue  
New York, New York 10154  
Attention: General Counsel  
Fax: (212) 546-9562

With a copy to:

Bristol-Myers Squibb Pharmaceutical Group  
Route 206 and Province Line Road  
Princeton, NJ 08543-4000  
Attention: P. Joseph Campisi, Jr.  
Email: joseph.campisi@bms.com

And a further copy (which shall not constitute notice to the Purchaser) to:

Kirkland & Ellis LLP  
601 Lexington Avenue  
New York, NY 10022  
Attention: Daniel Wolf and Douglas Ryder  
Email: daniel.wolf@kirkland.com; douglas.ryder@kirkland.com

or such other address or electronic mail address as the Company or Purchaser may designate by ten (10) days advance written notice to the other parties hereto.

**7.12 Expenses; Taxes.** Each party shall pay all costs and expenses that it incurs with respect to the negotiation, execution, delivery and performance of the Agreement

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**7.13 Attorneys' Fees.** In the event that any Action is instituted under or in relation to this Agreement, including without limitation to enforce any provision in this Agreement, the prevailing party in such dispute shall be entitled to recover from the losing party all fees, costs and expenses of enforcing any right of such prevailing party under or with respect to this Agreement, including without limitation, such reasonable fees and expenses of attorneys and accountants, which shall include, without limitation, all fees, costs and expenses of appeals.

**7.14 Titles and Subtitles.** The titles of the sections and subsections of the Agreement are for convenience of reference only and are not to be considered in construing this Agreement.

**7.15 Counterparts.** This Agreement may be executed in any number of counterparts (including via facsimile, PDF or other electronic signature), each of which shall be an original, but all of which together shall constitute one instrument.

**7.16 Broker’s Fees.** Each party hereto represents and warrants that no agent, broker, investment banker, person or firm acting on behalf of or under the authority of such party hereto is or will be entitled to any broker’s or finder’s fee or any other commission directly or indirectly in connection with the transactions contemplated herein. Each party hereto further agrees to indemnify each other party for any claims, losses or expenses incurred by such other party as a result of the representation in this Section 7.16 being untrue.

**7.17 Pronouns.** All pronouns contained herein, and any variations thereof, shall be deemed to refer to the masculine, feminine or neutral, singular or plural, as to the identity of the parties hereto may require. The words “include,” “includes” and “including” will be deemed to be followed by the phrase “without limitation”. The meanings given to terms defined herein will be equally applicable to both the singular and plural forms of such terms. All references to “dollars” or “\$” will be deemed references to the lawful money of the United States of America. All Exhibits attached hereto and all other attachments hereto are hereby incorporated herein by reference and made a part hereof.

**7.18 Third Party Beneficiaries.** None of the provisions of this Agreement shall be for the benefit of or enforceable by any Third Party, including any creditor of any party hereto. No 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 any party hereto.

**7.19 No Strict Construction.** This Agreement has been prepared jointly and will not be construed against either party. In the event an ambiguity or question of intent or interpretation arises, this Agreement shall be construed as if drafted jointly by the parties hereto, and no presumption or burden of proof shall arise favoring or disfavoring any party hereto by virtue of the authorship of any provisions of this Agreement.

[Signature Page to Follow]

IN WITNESS WHEREOF, the parties hereto have executed this Agreement as of the date set forth in the first paragraph hereof.

**Company:**

**UNIQUIRE N.V.**

By: \_\_\_\_\_  
Name:  
Title:

[Signature Page to the uniQure N.V. Share Subscription Agreement]

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IN WITNESS WHEREOF, the parties hereto have executed this Agreement as of the date set forth in the first paragraph hereof.

**Purchaser:**

**Bristol-Myers Squibb Company**

By: \_\_\_\_\_  
Name:  
Title:

[Signature Page to the uniQure N.V. Share Subscription Agreement]

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**EXHIBIT A**

Investor Agreement

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**EXHIBIT B**

Warrant Agreements

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

EXECUTION VERSION

## INVESTOR AGREEMENT

By and Between

BRISTOL-MYERS SQUIBB COMPANY

and

UNIQUE N.V.

Dated as of April 6, 2015

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## UNIQUE N.V.

### INVESTOR AGREEMENT

THIS INVESTOR AGREEMENT (this “**Agreement**”) is made as of April 6, 2015, by and between Bristol-Myers Squibb Company, a Delaware corporation (“**Investor**”), and uniQure N.V., a public company with limited liability (*naamloze vennootschap*) incorporated under the laws of the Netherlands (the “**Company**”).

WHEREAS, the Share Subscription Agreement, dated as of the date hereof, by and between the Investor and the Company (the “**Subscription Agreement**”) provides for the issuance and sale by the Company to the Investor, and the subscription by the Investor, of a number of the Company’s ordinary shares, par value €0.05 per share (the “**Ordinary Shares**”);

WHEREAS, as a condition to consummating the transactions contemplated by the Subscription Agreement, the Investor and the Company have agreed upon certain rights and restrictions as set forth herein with respect to the Purchased Shares and other securities of the Company Beneficially Owned by the Investor and its Affiliates, and it is a condition to the closings under the Subscription Agreement that this Agreement be executed and delivered by the Investor and the Company;

WHEREAS, simultaneously with the execution of the Subscription Agreement, the Company and the Investor entered into the Collaboration Agreement, and

WHEREAS, as of the date hereof, the Company and the Investor entered into the Seventh Target Warrant Agreement (the “**Seventh Target Warrant Agreement**”) and the Tenth Target Warrant Agreement (the “**Tenth Target Warrant Agreement**”) and together with the Seventh Target Warrant Agreement, the “**Warrant Agreements**”);

NOW, THEREFORE, in consideration of the premises and mutual agreements hereinafter set forth, and for other valuable consideration, the receipt and sufficiency of which are hereby acknowledged, the parties hereto agree as follows:

**1. Definitions.** As used in this Agreement, the following terms shall have the following meanings:

“**Affiliate**” shall mean, with respect to any Person, another Person which controls, is controlled by or is under common control with such Person. A Person shall be deemed to control another Person if such Person possesses, directly or indirectly, the power to direct or cause the direction of the management and policies of such Person, whether through the ownership of voting securities, by contract or otherwise. Without limiting the generality of the foregoing, a Person shall be deemed to control another Person if any of the following conditions is met: (i) 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 (ii) 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. For the purposes of this Agreement, in no event shall the Investor or any of its Affiliates be deemed

Affiliates of the Company or any of its Affiliates, nor shall the Company or any of its Affiliates be deemed Affiliates of the Investor or any of its Affiliates.

“**Agreement**” has the meaning ascribed to such term in the introductory paragraph to this Agreement

“**Beneficial Owner**,” “**Beneficially Owns**,” “**Beneficial Ownership**” and terms of similar import used in this Agreement shall, with respect to a Person, have the meaning set forth in Rule 13d-3 under the Exchange Act (i) assuming the full conversion into, and exercise and exchange for, Ordinary Shares of all Ordinary Share Equivalents beneficially owned by such Person and (ii) determined without regard for the number of days in which such Person has the right to acquire such beneficial ownership.

“**Business Day**” means any day except Saturday, Sunday and any day on which banking institutions in New York, New York and Amsterdam, the Netherlands generally are closed as a result of federal, state or local holiday.

“**Change of Control**” shall mean, with respect to a Person, any of the following events: (i) any Person is or becomes the Beneficial Owner (except that a Person shall be deemed to have Beneficial Ownership of all shares that any such Person has the right to acquire, whether such right which may be exercised immediately or only after the passage of time), directly or indirectly, of a majority of the total voting power represented by all shares of such Person’s outstanding capital stock; (ii) such Person consolidates with or merges into another corporation or entity, or any corporation or entity consolidates with or merges into such Person, other than (A) a merger or consolidation which would result in the voting securities of such Person outstanding immediately prior to such merger or consolidation continuing to represent (either by remaining outstanding or by being converted into voting securities of the surviving entity or any parent thereof) a majority of the combined voting power of the voting securities of such Person or such surviving entity or any parent thereof outstanding immediately after such merger or consolidation, or (B) a merger or consolidation effected to implement a recapitalization of such Person (or similar transaction) in which no Person becomes the Beneficial Owner, directly or indirectly, of a majority of the total voting power of all shares of capital stock of such Person, or (iii) such Person conveys, transfers or leases all or substantially all of its assets, to any Person other than a wholly owned Affiliate of such Person.

“**Collaboration Agreement**” shall mean the Collaboration and License Agreement, of even date with the Subscription Agreement, between the Investor and uniQure Biopharma B.V., an Affiliate of the Company.

**“Company”** has the meaning ascribed to such term in the introductory paragraph to this Agreement.

**“Disposition”** or **“Dispose of”** shall mean any (i) offer, pledge, sale, contract to sell, sale of any option or contract to purchase, purchase of any option or contract to sell, grant of any option, right or warrant for the sale of, or other disposition of or transfer of any Ordinary Shares, or any Ordinary Share Equivalents, including, without limitation, any “short sale” or similar arrangement, or (ii) swap or any other agreement or any transaction that transfers, in whole or in

part, directly or indirectly, the economic consequence of ownership of Ordinary Shares, whether any such swap or transaction is to be settled by delivery of securities, in cash or otherwise.

**“Exchange Act”** shall mean the U.S. Securities Exchange Act of 1934, as amended, and the rules and regulations of the SEC promulgated thereunder.

**“Filing Date”** shall mean as soon as practicable, but in no event later than (i) with respect to any Registration Statement to be filed on Form F-1 (or any applicable successor form), sixty (60) days after receipt by the Company of a Demand Request for such Registration Statement and (ii) with respect to any Registration Statement to be filed on Form F-3 (or any applicable successor form), thirty (30) days after receipt by the Company of a Demand Request for such Registration Statement.

**“Governmental Authority”** shall mean any court, agency, authority, department, regulatory body or other instrumentality of any government or country or of any national, federal, state, provincial, regional, county, city or other political subdivision of any such government or country or any supranational organization of which any such country is a member.

**“Holders”** shall mean (but, in each case, only for so long as such Person remains an Affiliate of the Investor) the Investor and any Permitted Transferee thereof, if any, in accordance with Section 2.11.

**“IFRS”** shall mean the International Financial Reporting Standards as issued by the International Accounting Standards Board.

**“Initial Closing Date”** shall have the meaning set forth in the Subscription Agreement.

**“Initial Share Purchase Price”** shall mean the price per share paid by the Investor for the Initial Shares (as defined in the Subscription Agreement).

**“Investor”** has the meaning ascribed to such term in the introductory paragraph to this Agreement.

**“Law”** or **“Laws”** shall mean all laws, statutes, rules, regulations, orders, judgments, injunctions and/or ordinances of any Governmental Authority.

**“Lock-up Term”** shall, in respect of each Ordinary Share comprising the Lock-Up Securities, mean the period from and after the date of this Agreement until the occurrence of the relevant event set forth in Section 6.3.

**“Ordinary Shares”** has the meaning ascribed to such term in the Recitals of this Agreement.

**“Ordinary Share Equivalents”** shall mean any options, warrants or other securities or rights convertible into or exercisable or exchangeable for, whether directly or following conversion into or exercise or exchange for other options, warrants or other securities or rights,

Ordinary Shares or any swap, hedge or similar agreement or arrangement that transfers in whole or in part, the economic risk of ownership of, or voting or other rights of, the Ordinary Shares.

**“Other Holders”** shall mean any Person having rights to participate in a registration of the Company’s securities.

**“Outstanding Ordinary Shares”** shall mean, at any time, the issued and outstanding Ordinary Shares at such time, as well as all share capital issued and outstanding as a result of any share split, share dividend, or reclassification of Ordinary Shares distributable, on a pro rata basis, to all holders of Ordinary Shares.

**“Permitted Transferee”** shall mean a directly or indirectly controlled Affiliate of the Investor, or the acquiring Person in the case of a Change of Control of the Investor.

**“Person”** shall mean any individual, partnership, firm, corporation, association, trust, unincorporated organization, government or any department or agency thereof or other entity, as well as any syndicate or group that would be deemed to be a Person under Section 13(d)(3) of the Exchange Act.

**“Prospectus”** shall mean the prospectus forming a part of any Registration Statement, as supplemented by any and all prospectus supplements and as amended by any and all amendments (including post-effective amendments) and including all material incorporated by reference or explicitly deemed to be incorporated by reference in such prospectus.

**“Purchased Shares”** shall mean any Ordinary Shares acquired by the Investor pursuant to the Subscription Agreement or the Warrant Agreements, and shall be adjusted for (i) any share split, share dividend, share exchange, merger, consolidation or similar recapitalization and (ii) any Ordinary Shares issued as (or issuable upon the exercise of any warrant, right or other security that is issued as) a dividend or other distribution with respect to, or in exchange or in replacement of, the Purchased Shares.



“**registers,**” “**registered,**” and “**registration**” refer to a registration effected by preparing and filing a Registration Statement or similar document in compliance with the Securities Act, and the declaration or ordering of effectiveness of such Registration Statement or document by the SEC.

“**Registrable Securities**” shall mean the Purchased Shares and any additional Ordinary Shares acquired by the Purchaser in compliance with the limitations set forth in Section 3.1(a), excluding in all cases, however, (i) any Registrable Securities if and after they have been transferred to a Permitted Transferee in a transaction in connection with which registration rights granted hereunder are not assigned, or (ii) any Registrable Securities sold to or through a broker or dealer or underwriter in a public distribution or a public securities transaction.

“**Registration Expenses**” shall mean all registration expenses incurred in connection with any Required Registration pursuant to Section 2.1 or 2.7, including, without limitation, all registration and filing fees, fees and expenses of compliance with securities or blue sky Laws (including reasonable fees and disbursements of counsel in connection with blue sky qualifications of any Registrable Securities), processing, duplicating and printing expenses,

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internal expenses of the Company (including, without limitation, all salaries and expenses of its officers and employees performing legal or accounting duties), fees and expenses incurred in connection with the listing of the Purchased Shares to be registered on each securities exchange, if any, on which equity securities issued by the Company are then listed or the quotation of such securities on any national securities exchange on which equity securities issued by the Company are then quoted, fees and disbursements of counsel for the Company and its independent certified public accountants (including the expenses of any management review, reasonable fees and disbursements of counsel for the Investor (up to a maximum of \$[\*\*]), cold comfort letters or any special audits required by or incident to such performance and compliance), Securities Act liability insurance (if the Company elects to obtain such insurance), the reasonable fees and expenses of any special experts retained by the Company in connection with such registration, and fees and expenses of other Persons retained by the Company. Notwithstanding the foregoing, no Selling Expenses shall be Registration Expenses.

“**Registration Rights Term**” shall mean the period from and after the expiration of the Lock-up Term until the occurrence of any event set forth in Section 6.1.

“**Registration Statement**” shall mean any registration statement of the Company under the Securities Act that covers any of the Registrable Securities pursuant to the provisions of this Agreement, including the related Prospectus, all amendments and supplements to such registration statement (including post-effective amendments), and all exhibits and all materials incorporated by reference or explicitly deemed to be incorporated by reference in such Registration Statement.

“**Required Period**” with respect to a Required Registration shall mean the earlier of (i) the date on which all Registrable Securities covered by such Required Registration are sold pursuant thereto and (ii) [\*\*] days following the first day of effectiveness of the Registration Statement for such Required Registration, in each case subject to extension as set forth herein; provided, however, that in no event will the Required Period expire prior to the expiration of the applicable period referred to in Section 4(3) of the Securities Act and Rule 174 promulgated thereunder; provided, further, however, that (i) such one-hundred twenty (120) day period shall be extended for a period of time equal to the period the Holder refrains, at the request of an underwriter of Ordinary Shares (or other securities) of the Company, from selling any securities included in such registration, and (ii) in the case of any registration of Registrable Securities on Form F-3 that are intended to be offered on a continuous or delayed basis, subject to compliance with applicable SEC rules, such [\*\*] day period shall be extended, if necessary, to keep the Registration Statement effective until the earlier of such time as all such Registrable Securities registered on such Registration Statement are sold.

“**Required Registration Cap**” shall mean two Required Registrations; plus one additional Required Registration following issuance of Ordinary Shares pursuant to the full exercise of the Seventh Target Warrant Agreement or the Tenth Target Warrant Agreement.

“**SEC**” shall mean the United States Securities and Exchange Commission.

“**Second Closing Date**” shall have the meaning set forth in the Subscription Agreement.

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“**Securities Act**” shall mean the Securities Act of 1933, as amended, and the rules and regulations of the SEC promulgated thereunder.

“**Selling Expenses**” shall mean all underwriting discounts and selling commissions applicable to the sale of Registrable Securities pursuant to this Agreement.

“**Standstill Term**” shall mean the period from and after the date of this Agreement until the occurrence of any event set forth in Section 6.2.

“**Subscription Agreement**” has the meaning ascribed to such term in the Recitals of this Agreement.

“**Third Party**” shall mean any Person other than the Investor, the Company or any of their respective Affiliates.

“**Trading Day**” means a day on which the Trading Market is open for trading.

“**Trading Market**” shall mean The NASDAQ Stock Market or New York Stock Exchange to the extent that the Ordinary Shares are then listed on such exchange, as applicable.

“**Transaction Agreements**” shall mean this Agreement, the Warrant Agreements, the Collaboration Agreement and the Subscription Agreement.

“**Underwritten Registration**” or “**Underwritten Offering**” shall mean a registration in which Registrable Securities are sold to an underwriter for reoffering to the public.

“**Voting Agreement Term**” shall mean the period from and after the date of this Agreement until the occurrence of the event set forth in Section 6.4.

“VWAP” shall mean, for any date, the price determined by the first of the following clauses that applies: (a) if the Ordinary Shares are then listed or quoted on a Trading Market, the daily volume weighted average price of the Ordinary Shares for such date (or the nearest preceding date) on the Trading Market on which the Ordinary Shares are then listed or quoted as reported by Bloomberg L.P. (based on a Trading Day from 9:30 a.m. (New York City time) to 4:02 p.m. (New York City time)) or (b) in all other cases, the fair market value of a share of Ordinary Shares as determined by an independent appraiser selected in good faith and mutually agreed upon between the Company and the Investor, the fees and expenses of which shall be paid by the Company.

“Warrant Agreements” has the meaning ascribed to such term in the Recitals of this Agreement.

## 2. Registration Rights.

**2.1 Required Registration.** If, during the Registration Rights Term, the Company receives from any Holder or Holders a written request or requests (each, a “Demand Request”) that the Company file a Registration Statement under the Securities Act to effect the registration (a “Required Registration”) of Registrable Securities, the Company shall use all

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reasonable best efforts to file a Registration Statement covering such Holders’ Registrable Securities as soon as practicable (and by the applicable Filing Date) and shall use all reasonable best efforts to, as soon as practicable thereafter, effect the registration of the Registrable Securities to permit or facilitate the sale and distribution of all or such portion of such Holder’s or Holders’ Registrable Securities as are specified in such Demand Request, subject however, to the conditions and limitations set forth herein; provided, however, that the Company shall not be obligated to effect any registration of Registrable Securities upon receipt of a Demand Request pursuant to this Section 2.1 if:

(a) the Company has already completed a number of Required Registrations equal to the then applicable Required Registration Cap;

(b) the market value of the Registrable Securities proposed to be included in the registration is less than [\*\*], calculated by multiplying such number of Registrable Securities by the VWAP on the date of a Registration Demand pursuant to this Section 2.1; provided, however, that, in the event that the market value of all Registrable Securities then held by the Holders is less than [\*\*], such Holders shall be able to make a Demand Request, and the Company shall be obligated pursuant to the terms hereof to comply with such Demand Request provided that such Demand Request apply to all of the Registrable Securities held by the Holders at the time of such Demand Request;

(c) the Company furnishes to the Holders a certificate signed by an authorized officer of the Company stating that (i) within [\*\*] days of receipt of the Demand Request under this Section 2.1, the Company expects to file a registration statement for the public offering of securities for the account of the Company (other than a registration of securities (A) issuable pursuant to an employee share option, share purchase or similar plan, (B) issuable pursuant to a merger, exchange offer or a transaction of the type specified in Rule 145(a) under the Securities Act or (B) in which the only securities being registered are securities issuable upon conversion of debt securities which are also being registered), provided, that the Company is actively employing good faith efforts to cause such registration statement to become effective, or (ii) the Company is engaged in a material transaction or has an undisclosed material corporate development, in either case, which would be required to be disclosed in the Registration Statement, and in the good faith judgment of the Company’s supervisory board, such disclosure would be detrimental to the Company and its shareholders at such time (in which case, the Company shall disclose the matter as promptly as reasonably practicable and thereafter file the Registration Statement, and each Holder agrees not to disclose any information about such material transaction to Third Parties until such disclosure has occurred or such information has entered the public domain other than through breach of this provision by such Holder), provided, however, that the Company shall have the right to only defer the filing of the Registration Statement pursuant to this subsection twice in any twelve (12) month period and, such deferral may not exceed a period of more than ninety (90) days in the aggregate after receipt of a Demand Request; or

(d) the Company has, within the twelve (12) month period preceding the date of the Demand Request, already effected one (1) Required Registration for any Holder pursuant to this Section 2.1.

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**2.2 Company Registration.** Effective from the expiration of the Lock-up Term, the Company shall notify the Holders in writing at least twenty (20) days prior to the filing of any Registration Statement (“Registration Notice”) and will afford each Holder an opportunity, subject to the terms and conditions of this Agreement, to include in such Registration Statement the number of Registrable Securities then held by such Holder that such Holder wishes to include in such Registration Statement. Each Holder desiring to include in any such Registration Statement all or any part of the Registrable Securities held by such Holder shall, within ten (10) days after receipt of the Registration Notice, so notify the Company in writing, and in such notification, inform the Company of the number of Registrable Securities such Holder wishes to include in such Registration Statement. If a Holder decides not to include Registrable Securities in any Registration Statement thereafter filed by the Company, such Holder shall nevertheless continue to have the right to include such Registrable Securities in any subsequent Registration Statement or Registration Statements as may be filed by the Company with respect to offerings of its securities (either by the Company or by its shareholders), all upon the terms and conditions set forth herein. Each Holder shall keep confidential and not disclose to any Third Party (i) its receipt of any Registration Notice and (ii) any information regarding the proposed offering as to which such notice is delivered, except as required by law, regulation or as compelled by subpoena. If a registration pursuant to this Section 2.2 is an Underwritten Offering, the right of any such Holder to include Registrable Securities in such registration shall be conditioned upon such Holder’s participation in such underwriting and the inclusion of such Holder’s Registrable Securities in the underwriting to the extent provided herein. The Company and all Holders proposing to distribute their Registrable Securities through such underwriting shall enter into an underwriting agreement in customary form with the managing underwriter or underwriters selected for such underwriting. Notwithstanding any other provision of this Section 2, if the managing underwriter for the Underwritten Offering determines in good faith that marketing factors require a limitation of the number of shares of Registrable Securities to be included in such Underwritten Offering and advises the Holders of such determination in writing, then the managing underwriter may exclude shares (including up to one hundred percent (100%) of the Registrable Securities) from the registration and the underwriting, with the number of Registrable Securities, if any, included in the registration and the underwriting being allocated to each of the Holders requesting inclusion of their Registrable Securities in such Registration Statement and all other Persons selling Ordinary Shares pursuant to such Registration Statement on a pro rata basis based on the total number of Ordinary Shares then held by each such Holder or other shareholder. Notwithstanding the foregoing, the Company shall have the right to terminate or withdraw any

registration initiated by it under this Section 2.2 prior to the effectiveness of such registration whether or not any Holder has elected to include securities in such registration.

**2.3 Underwritten Registration; Priority in Underwritten Offering.** If, pursuant to Section 2.1, the Holders intend to distribute all or any portion of the Registrable Securities covered by their request by means of an underwriting, the Holders shall so advise the Company as a part of their request made pursuant to Section 2.1. The majority in interest of the Holders initiating the Required Registration hereunder shall select the underwriter(s) for such offering, subject to the approval of the Company, which approval shall not be unreasonably withheld or delayed. If an offering of Registrable Securities registered pursuant to Section 2.1 is an Underwritten Offering, the right of any Holder to include its Registrable Securities in the Underwritten Offering shall be conditioned upon such Holder's participation in such

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Underwritten Offering and the inclusion of such Holder's Registrable Securities to the extent provided herein. All Holders requesting the inclusion of their Registrable Securities in such Underwritten Offering shall (together with the Company as provided in Section 2.7(i)) enter into an underwriting agreement in customary form with the underwriter or underwriters selected for such Underwritten Offering. Notwithstanding any other provision of this Section 2, if the managing underwriter for such Underwritten Offering determines in good faith that marketing factors require a limitation of the number of shares of Registrable Securities to be included in such Underwritten Offering and advises the Holders of such determination in writing, then the Company shall so advise all Holders that requested inclusion of their Registrable Securities in such Underwritten Offering, and the number of shares of Registrable Securities that may be included in such Underwritten Offering shall be allocated among the Holders in proportion (as nearly as practicable) to the amount of Registrable Securities of the Company owned by each Holder; provided, however, that the number of shares of Registrable Securities to be included in such Underwritten Offering shall not be reduced unless all other securities are first entirely excluded from such Underwritten Offering. In the event the Company advises the Holders of its intent to decrease the total number of Registrable Securities that may be included by the Holders in such Required Registration such that the number of Registrable Securities included in such Required Registration would be less than seventy-five percent (75%) of all Registrable Securities that the Holders requested be included in such Required Registration, then Holders representing a majority of the Registrable Securities requested to be included in such Required Registration will have the right to withdraw, on behalf of all Holders of all Registrable Securities requested to be so included, such Required Registration, in which case, such Required Registration will not count as a Required Registration for the purposes of Section 2.1(a), and the Company shall bear all Registration Expenses in connection therewith.

**2.4 Priority in Underwritten Required Registration.** With respect to any Required Registration of Registrable Securities requested pursuant to Section 2.1 that is an Underwritten Offering, the Company may also (i) propose to sell Ordinary Shares on its own behalf and (ii) provide written notice of such Required Registration to Other Holders and permit all such Other Holders who request to be included in the Required Registration to include any or all Company securities held by such Other Holders in such Required Registration on the same terms and conditions as the Registrable Securities. If the managing underwriter or underwriters of the Underwritten Offering to which any Required Registration relates advise the Company and the Holders of Registrable Securities that, in its good faith determination, the total amount of securities that such Holders, Other Holders, and the Company intend to include in such Required Registration is in an amount in the aggregate that would adversely affect the success of such Underwritten Offering, then such Required Registration shall include (i) first, all Registrable Securities of the Holders allocated, if the amount is less than all the Registrable Securities requested to be sold, pro rata on the basis of the total number of Registrable Securities held by such Holders; and (ii) second, as many other securities proposed to be included in the Required Registration by the Company and any Other Holders, allocated pro rata among the Company and such Other Holders, on the basis of the amount of securities requested to be included therein by the Company and each such Other Holder so that the total amount of securities to be included in such Underwritten Offering is the full amount that, in the written opinion of such managing underwriter, can be sold without materially and adversely affecting the success of such Underwritten Offering.

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**2.5 Effective Required Registrations.** A Required Registration will not be deemed to be effected for purposes of Section 2.1(a) if the Registration Statement for such Required Registration has not been declared effective by the SEC or become effective in accordance with the Securities Act and the rules and regulations thereunder and kept effective for the Required Period. In addition, if after such Registration Statement has been declared or becomes effective, (i) the offering of Registrable Securities pursuant to such Registration Statement is interfered with by any stop order, injunction, or other order or requirement of the SEC or other governmental agency or court such that the continued offer and sale of Registrable Securities being offered pursuant to such Registration Statement would violate applicable Law and such stop order, injunction or other order or requirement of the SEC or other governmental agency or court does not result from any act or omission of any Holder whose Registrable Securities are registered pursuant to such Registration Statement (an "**Interference**") and (ii) any such Interference is not cured within sixty (60) days thereof, such Required Registration will be deemed not to have been effected and will not count as a Required Registration. In the event such Interference occurs and is cured, the Required Period relating to such Registration Statement will be extended by the number of days of such Interference, including the date such Interference is cured.

**2.6 Continuous Effectiveness of Registration Statement.** The Company will use all reasonable best efforts to cause each Registration Statement filed pursuant to this Section 2 to be declared effective by the SEC or to become effective under the Securities Act as promptly as practicable and to keep each such Registration Statement that has been declared or becomes effective continuously effective for the Required Period.

**2.7 Obligations of the Company.** Whenever required under Section 2.1 to effect the registration of any Registrable Securities, the Company shall, as expeditiously as reasonably possible:

(a) prepare and file with the SEC a Registration Statement with respect to such Registrable Securities sought to be included therein; provided that at least five (5) Trading Days prior to filing any Registration Statement or Prospectus or any amendments or supplements thereto, the Company shall furnish to the Holders of the Registrable Securities covered by such Registration Statement, their counsel and the managing underwriter and their counsel copies of all such documents proposed to be filed, and any such Holder, managing underwriter or their respective counsel shall have the opportunity to comment on any information that is contained therein and the Company shall make the corrections reasonably requested by such Holder or the managing underwriter with respect to such information prior to filing any such Registration Statement or amendment;

(b) prepare and file with the SEC such amendments and post-effective amendments to any Registration Statement and any Prospectus used in connection therewith as may be necessary to keep such Registration Statement effective for the Required Period, and cause the Prospectus to be supplemented by any required prospectus supplement, and as so supplemented to be filed pursuant to Rule 424 under the Securities Act, to comply with

supplements thereto, the Company shall furnish to the Holders of the Registrable Securities covered by such Registration Statement, their counsel and the managing underwriter and their counsel copies of all such documents proposed to be filed, and any such Holder, managing underwriter or their respective counsel shall have the opportunity to comment on any information that is contained therein and the Company shall make the corrections reasonably requested by such Holder and the managing underwriter with respect to such information prior to filing any such Registration Statement or amendment;

(c) furnish to the Holders of Registrable Securities covered by such Registration Statement and the managing underwriter such numbers of copies of such Registration Statement, each amendment and supplement thereto, the Prospectus included in such Registration Statement (including each preliminary prospectus or free writing prospectus) in conformity with the requirements of the Securities Act, and such other documents as they may reasonably request in order to facilitate the disposition of Registrable Securities owned by them;

(d) notify the Holders of Registrable Securities covered by such Registration Statement, promptly after the Company shall receive notice thereof, of the time when such Registration Statement becomes or is declared effective or when any amendment or supplement or any Prospectus forming a part of such Registration Statement has been filed;

(e) notify the Holders of Registrable Securities covered by such Registration Statement promptly of any request by the SEC for the amending or supplementing of such Registration Statement or Prospectus or for additional information and promptly deliver to such Holders copies of any comments received from the SEC;

(f) notify the Holders promptly of any stop order suspending the effectiveness of such Registration Statement or Prospectus or the initiation of any proceedings for that purpose, and use all reasonable best efforts to obtain the withdrawal of any such order or the termination of such proceedings;

(g) use all reasonable best efforts to keep each Registration Statement continuously effective during the period such Registration Statement is required to remain effective pursuant to the terms of this Agreement;

(h) use all reasonable best efforts to register and qualify the Registrable Securities covered by such Registration Statement under such other securities or blue sky Laws of such jurisdictions as shall be reasonably requested by the Holders, use all reasonable best efforts to keep each such registration or qualification effective, including through new filings, or amendments or renewals, during the Required Period, and notify the Holders of Registrable Securities covered by such Registration Statement of the receipt of any written notification with respect to any suspension of any such qualification; provided, however, that the Company shall not be required in connection therewith or as a condition thereto to qualify to do business or to file a general consent to service of process in any such states or jurisdictions, unless the Company is already subject to service in such jurisdiction and except as may be required by the Securities Act;

(i) in the event of any Underwritten Offering, enter into and perform its obligations under an underwriting agreement, in usual and customary form, with the managing underwriter of the Underwritten Offering pursuant to which such Registrable Securities are being offered and take such other actions as are prudent and reasonably required in order to expedite or facilitate the disposition of such Registrable Securities, including causing its officers to participate in "road shows" and other information meetings organized by the managing underwriters;

(j) use all reasonable best efforts to obtain: (A) at the time of effectiveness of the Registration Statement covering such Registrable Securities, a "cold comfort letter" from the Company's independent certified public accountants covering such matters of the type customarily covered by "cold comfort letters" as the underwriters may reasonably request; and (B) at the time of any underwritten sale pursuant to such Registration Statement, a "bring-down comfort letter," dated as of the date of such sale, from the Company's independent certified public accountants covering such matters of the type customarily covered by "bring-down comfort letters" as the underwriters may reasonably request;

(k) promptly notify each Holder of Registrable Securities covered by such Registration Statement at any time when a Prospectus relating thereto is required to be delivered under the Securities Act of the happening of any event as a result of which the Prospectus included in such Registration Statement or any offering memorandum or other offering document includes an untrue statement of a material fact or omits to state any material fact required to be stated therein or necessary to make the statements therein not misleading in the light of the circumstances then existing, and promptly prepare a supplement or amendment to such Prospectus or file any other required document so that, as thereafter delivered to the purchasers of such Registrable Securities, such Prospectus will not contain an untrue statement of material fact or omit to state any fact necessary to make the statements therein not misleading;

(l) permit any Holder of Registrable Securities covered by such Registration Statement, which Holder in its reasonable judgment could reasonably be deemed to be an underwriter with respect to the Underwritten Offering pursuant to which such Registrable Securities are being offered, or to be a controlling Person of the Company, to reasonably participate in the preparation of such Registration Statement and to require the insertion therein of information to the extent concerning such Holder, furnished to the Company in writing, which in the reasonable judgment of such Holder and its counsel should be included;

(m) in connection with any Underwritten Offering, use all reasonable best efforts to obtain an opinion or opinions addressed to the underwriter or underwriters in customary form and scope from counsel for the Company;

(n) upon reasonable notice and during normal business hours, subject to the Company receiving customary confidentiality undertakings or agreements from any Holder of Registrable Securities covered by such Registration Statement or other Person obtaining access to Company records, documents, properties or other information pursuant to this subsection (n), make available for inspection by a representative of such Holder and any

pertinent corporate documents and properties of the Company, and use all reasonable best efforts to cause the supervisory and management boards and employees of the Company to supply all information reasonably requested by any such representative, underwriter, attorneys or accountants in connection with the Registration Statement;

(o) use all reasonable best efforts to comply with all applicable rules and regulations of the SEC relating to such registration and make generally available to its security holders earning statements satisfying the provisions of Section 11(a) of the Securities Act, provided that the Company will be deemed to have complied with this subsection (o) with respect to such earning statements if it has satisfied the provisions of Rule 158;

(p) if requested by the managing underwriter or any selling Holder, promptly incorporate in a prospectus supplement or post-effective amendment such information as the managing underwriter or any selling Holder reasonably requests to be included therein, with respect to the Registrable Securities being sold by such selling Holder, including, without limitation, the purchase price being paid by the underwriters and with respect to any other terms of the Underwritten Offering of Registrable Securities to be sold in such offering, and promptly make all required filings of such prospectus supplement or post-effective amendment;

(q) cause the Registrable Securities covered by such Registration Statement to be listed on each securities exchange, if any, on which equity securities issued by the Company are then listed;

(r) reasonably cooperate with each selling Holder and each underwriter participating in the disposition of such Registrable Securities and their respective counsel in connection with filings required to be made with the Financial Industry Regulatory Authority, Inc., if any; and

(s) use reasonable best efforts to take all other steps reasonably necessary to effect the registration of the Registrable Securities contemplated hereby.

**2.8 Furnish Information.** A Holder shall furnish to the Company such information regarding itself and the Registrable Securities held by it, pursuant to this Section 2 with respect to the Registrable Securities of any such selling Holder, as shall be reasonably necessary to effect the registration of such Holder's Registrable Securities.

**2.9 Expenses.** Except as specifically provided herein, all Registration Expenses incurred in connection with any registration hereunder shall be borne by the Company and all Selling Expenses incurred in connection with any registration hereunder shall be borne by the Holders of Registrable Securities covered by a Registration Statement, pro rata on the basis of the number of Registrable Securities registered on their behalf in such Registration Statement.

**2.10 Indemnification.** In the event any Registrable Securities are included in a Registration Statement under this Agreement:

(a) The Company shall indemnify and hold harmless each Holder including Registrable Securities in any such Registration Statement, any underwriter (as defined in the Securities Act) for such Holder and each Person, if any, who controls such Holder or

underwriter within the meaning of Section 15 of the Securities Act or Section 20 of Exchange Act and the officers, directors, owners, agents and employees of such controlling Persons, against any and all losses, claims, damages or liabilities (joint or several) to which they may become subject under any securities Laws including, without limitation, the Securities Act, the Exchange Act, or any other statute or common law of the United States or any other country or political subdivision thereof, or otherwise, including the amount paid in settlement of any litigation commenced or threatened (including any amounts paid pursuant to or in settlement of claims made under the indemnification or contribution provisions of any underwriting or similar agreement entered into by such Holder in connection with any offering or sale of securities covered by this Agreement), and shall promptly reimburse them, as and when incurred, for any legal or other expenses incurred by them in connection with investigating any claims and defending any actions, insofar as any such losses, claims, damages or liabilities (or actions in respect thereof) arise out of or are based upon any of the following statements, omissions or violations (each, a "**Violation**"): (i) any untrue statement or alleged untrue statement of a material fact contained in or incorporated by reference into such Registration Statement, including any preliminary prospectus or final prospectus contained therein or any free writing prospectus or any amendments or supplements thereto, or in any offering memorandum or other offering document relating to the offering and sale of such securities, (ii) the omission or alleged omission to state therein a material fact required to be stated therein, or necessary to make the statements therein not misleading or (iii) any violation or alleged violation by the Company (or any of its agents or Affiliates) of the Securities Act, the Exchange Act, any state securities Law, or any rule or regulation promulgated under any state securities Law; provided, however, the Company shall not be liable in any such case for any such loss, claim, damage, liability or action to the extent that it (A) arises out of or is based upon a Violation which occurs solely in reliance upon and in conformity with written information furnished expressly for use in connection with such registration by such Holder; or (B) is determined by a court of competent jurisdiction by final and nonappealable judgment that it is caused by such Holder's disposition of Registrable Securities during any period during which such Holder is obligated to discontinue any disposition of Registrable Securities as a result of any stop order suspending the effectiveness of any registration statement or prospectus with respect to Registrable Securities and the Company had timely complied with its obligation to notify the Holder in accordance with Section 2.7. The Company shall pay, as incurred, any legal or other expenses reasonably incurred by any Person intended to be indemnified pursuant to this Section 2.10(a), in connection with investigating or defending any such loss, claim, damage, liability or action; provided, however, that the indemnity agreement contained in this Section 2.10(a) shall not apply to amounts paid in settlement of any such loss, claim, damage, liability or action if such settlement is effected without consent of the Company, which consent shall not be unreasonably withheld.

(b) Each Holder including Registrable Securities in a registration statement shall indemnify and hold harmless the Company, each of its supervisory and management board members, each of its senior executives who has signed the registration statement, each Person, if any, who controls the Company within the meaning of Section 15 of the Securities Act or Section 20 of the Exchange Act and the officers, directors, owners, agents and employees of such controlling Persons, any underwriter, any other Holder selling securities in such registration statement and any controlling Person of

arise out of or are based upon any Violation, in each case to the extent (and only to the extent) that such Violation (i) arises out of or is based upon a Violation which occurs solely in reliance upon and in conformity with written information furnished expressly for use in connection with such registration by such Holder; or (ii) is caused by such Holder's disposition of Registrable Securities during any period during which such Holder is obligated to discontinue any disposition of Registrable Securities as a result of any stop order suspending the effectiveness of any registration statement or prospectus with respect to Registrable Securities and the Company had timely complied with its obligation to notify the Holder in accordance with Section 2.7; provided, however, that in no event shall any indemnity under this Section 2.10(b) payable by a Holder exceed the amount by which the net proceeds actually received by such holder from the sale of Registrable Securities included in such registration exceeds the amount of any other losses, expenses, settlements, damages, claims and liabilities that such holder has been required to pay by reason of such Violation. Each such Holder shall pay, as incurred, any legal or other expenses reasonably incurred by any Person intended to be indemnified pursuant to this Section 2.10(b), in connection with investigating or defending any such loss, claim, damage, liability or action; provided, however, that the indemnity agreement contained in this Section 2.10(b) shall not apply to amounts paid in settlement of any such loss, claim, damage, liability or action if such settlement is effected without consent of the Holder, which consent shall not be unreasonably withheld.

(c) Promptly after receipt by an indemnified party under this Section 2.10 of notice of the commencement of any action (including any action by a Governmental Authority), such indemnified party shall, if a claim in respect thereof is to be made against any indemnifying party under this Section 2.10, deliver to the indemnifying party a written notice of the commencement thereof and the indemnifying party shall have the right to participate in, and, to the extent the indemnifying party so desires, jointly with any other indemnifying party similarly noticed, to assume the defense thereof with counsel mutually satisfactory to the parties; provided, however, that an indemnified party (together with all other indemnified parties that may be represented without conflict by one counsel) shall have the right to retain its own counsel, with the reasonable fees and expenses to be paid by the indemnifying party, if representation of such indemnified party by the counsel retained by the indemnifying party would be inappropriate due to actual or potential differing interests between such indemnified party and any other party represented by such counsel in such proceeding. The failure to deliver written notice to the indemnifying party within a reasonable time of the commencement of any such action, if prejudicial to its ability to defend such action, shall relieve such indemnifying party of any liability to the indemnified party under this Section 2.10, but the omission so to deliver written notice to the indemnifying party shall not relieve it of any liability that it may have to any indemnified party otherwise than under this Section 2.10.

(d) In order to provide for just and equitable contribution to joint liability in any case in which a claim for indemnification is made pursuant to this Section 2.10 but it is judicially determined (by the entry of a final judgment or decree by a court of competent jurisdiction and the expiration of time to appeal or the denial of the last right of appeal) that such indemnification may not be enforced in such case notwithstanding the fact that this Section 2.10 provided for indemnification in such case, the Company and each Holder of Registrable Securities shall contribute to the aggregate losses, claims, damages or liabilities to which they may be subject (after contribution from others) in proportion to the relative fault of the

Company, on the one hand, and such Holder, severally, on the other hand; provided, however, that in any such case, no Person guilty of fraudulent misrepresentation (within the meaning of Section 11(f) of the Securities Act) shall be entitled to contribution from any Person who was not guilty of such fraudulent misrepresentation; provided further, however, that in no event shall any contribution under this Section 2.10(d) on the part of any Holder exceed the net proceeds received by such Holder from the sale of Registrable Securities giving rise to such contribution obligation, except in the case of willful misconduct or fraud by such Holder.

(e) The obligations of the Company and the Holders under this Section 2.10 shall survive the completion of any offering of Registrable Securities in a registration statement under this Agreement and otherwise.

**2.11 Assignment of Registration Rights.** The rights to cause the Company to register any Registrable Securities pursuant to this Agreement may be assigned in whole or in part (but only with all restrictions and obligations set forth in this Agreement) by a Holder to a Permitted Transferee which acquires Registrable Securities from such Holder; provided, however, (a) such Holder shall, within five (5) days prior to such transfer, furnish to the Company written notice of the name and address of such Permitted Transferee, details of its status as a Permitted Transferee and details of the Registrable Securities with respect to which such registration rights are being assigned, (b) the Permitted Transferee, prior to or simultaneously with such transfer or assignment, shall agree in writing to be subject to and bound by all restrictions and obligations set forth in this Agreement, (c) the Investor shall continue to be bound by all restrictions and obligations set forth in this Agreement and (d) such transfer or assignment shall be effective only if immediately following such transfer or assignment the further disposition of such Registrable Securities by the Permitted Transferee is restricted under the Securities Act and other applicable securities Law.

### **3. Restrictions on Beneficial Ownership.**

**3.1 Standstill.** During the Standstill Term, except for (i) as otherwise contemplated by the Transaction Agreements or Section 3.2 below, (ii) as the Company's supervisory board or management board shall otherwise specifically request in writing, or (iii) for any conversions, reclassifications, reorganizations, share dividends, share splits, reverse splits and similar events which occur with respect to the Ordinary Shares, neither the Investor nor any Permitted Transferee (collectively, the "**Standstill Parties**"), shall (and the Investor shall cause any Permitted Transferee, as applicable, not to), directly or indirectly, except as expressly approved or invited in writing by the Company:

(a) acquire Beneficial Ownership of Outstanding Ordinary Shares and/or Ordinary Share Equivalents, or make a tender, exchange or other offer to acquire Outstanding Ordinary Shares and/or Ordinary Share Equivalents such that after such acquisition of Ordinary Shares and/or Ordinary Share Equivalents, and conversion of such Ordinary Share Equivalents, if applicable, the Standstill Parties would collectively Beneficially Own more than (i) 4.9% of the Outstanding Ordinary Shares (as determined after giving effect to the Shares issued at the Initial Closing) after the Initial Closing Date but before the Second Closing Date, or (ii) more than 9.9% of the Outstanding Ordinary Shares (as determined after giving effect to Shares issued at the Second Closing) after the Second Closing Date but before the purchase of

Ordinary Shares pursuant to the Seventh Target Warrant Agreement, or (iii) more than 14.9% of the Outstanding Ordinary Shares (as determined after giving effect to the Ordinary Shares issued upon the exercise of the Seventh Target Warrant Agreement) after the purchase of Ordinary Shares pursuant to the Seventh Target Warrant Agreement but before the purchase of Ordinary Shares pursuant to the Tenth Target Warrant Agreement, or (iv) more than 19.9% of the Outstanding Ordinary Shares (as determined after giving effect to the Ordinary Shares issued upon the exercise of the Tenth Target Warrant Agreement) after the purchase of Ordinary Shares pursuant to the Tenth Target Warrant Agreement; provided, however, that (i) notwithstanding the provisions of this Section 3.1(a), if the number of Outstanding Ordinary Shares is reduced or if the aggregate ownership of the Standstill Parties is increased as a result of a repurchase by the Company of Outstanding Ordinary Shares, share split, share dividend or a recapitalization of the Company, the Standstill Parties shall not be required to dispose of any of their holdings of Outstanding Ordinary Shares even though such action resulted in the Standstill Parties' Beneficial Ownership increasing; and (ii) for purposes of clarification, the limitations set forth in this Section 3.1(a) shall in no way prohibit, restrict or limit the ability of the Standstill Parties to acquire Ordinary Shares on the open market or otherwise so long as, after giving effect to such acquisitions, the Standstill Parties do not hold in excess of the relevant percentage(s) of Ordinary Shares set forth above during the time periods set forth above;

(b) propose, offer or participate in any effort to acquire the Company or any of its subsidiaries or all or substantially all of the assets of the Company and its subsidiaries taken as a whole;

(c) propose, offer or participate in any tender offer, exchange offer, or other business combination or Change of Control transaction involving the Company or any of its subsidiaries, or any recapitalization, restructuring, liquidation, disposition, dissolution or other extraordinary corporate transaction involving the Company or any of its subsidiaries;

(d) seek to call, request the call of, or call an extraordinary general meeting of the shareholders of the Company, or make or seek to make a shareholder proposal at any general meeting of the shareholders of the Company, or make a request for a list of the Company's shareholders, or seek election to the supervisory board or seek to place a representative on the supervisory board, or seek the removal of any member from the supervisory board, or otherwise acting alone or in concert with others, seek to control or influence the governance or policies of the Company;

(e) solicit powers of attorney, proxies, designations or written consents of shareholders, or conduct any binding or nonbinding referendum with respect to Ordinary Shares, or make or in any way participate in any "solicitation" of any "proxy" (power of attorney) to vote any Ordinary Shares with respect to any matter, or become a participant in any contested solicitation for the election of members of the supervisory board with respect to the Company (as such terms are defined or used in the Exchange Act and the rules promulgated thereunder);

(f) make or issue or cause to be made or issued any public disclosure, announcement or statement (i) in support of any solicitation described in clause (e) above, (ii) in

support of any matter described in clause (d) above, or (iii) concerning any potential matter described in clause (c) above;

(g) form, join, or in any other way participate in, a "partnership, limited partnership, syndicate or other group" with respect to Ordinary Shares, or deposit any Ordinary Shares in a voting trust or similar arrangement, or subject any Ordinary Shares to any voting agreement or pooling arrangement, or grant any power of attorney with respect to any Purchased Shares;

(h) except as otherwise provided by applicable law, rule or regulation, publicly disclose, or cause or facilitate the public disclosure of, any intent, purpose, plan or proposal to obtain any waiver, consent under, or amendment of, any of these restrictions or bring any action or otherwise act to contest the validity or enforceability of these restrictions or seek a release from these restrictions or obligations; or

(i) enter into any discussions, negotiations, agreements or understandings with any Third Party with respect to the foregoing, or advise, assist, knowingly encourage, support, provide financing to or seek to persuade any Third Party to take any action with respect to any of the foregoing, or act in concert with others or as part of a group with respect to any of the foregoing.

### **3.2 Standstill Exceptions.** The restrictions contained in Section 3.1 shall not:

(a) limit the Investor's (or any of its Affiliates') ability to (i) inquire or make a request, orally or in writing, with respect to any amendment or waiver of any of the type described in Section 3.1 (including this paragraph (a)) or (ii) make or submit to the Company, the supervisory board of the Company or the management board of the Company at any time a bona fide non-public and confidential proposal relating to a business combination transaction or similar transaction so long as such action would not reasonably be expected to require the Company to make a public announcement relating thereto;

(b) prohibit the Investor nor any of its Affiliates from acquiring securities of the Company by or through (i) a diversified mutual or pension fund managed by an independent investment adviser or pension plan established for the benefit of Investors' or its Affiliates' employees, (ii) any of Investors' employee benefit plans or of its Affiliates for which investment decisions are made by an independent trustee or (iii) any stock portfolio not controlled or managed by the Investor or any of its affiliates which invest in the Company among other companies; provided, that the purpose of entering into such transaction is not to circumvent the terms in Section 3.1;

(c) prohibit the Investor or any of its Affiliates from acquiring securities of, or from entering into any merger or other business combination with, another company or other Person which Beneficially Owns securities of the Company; provided, that the purpose of entering into such transaction is not to circumvent the terms in Section 3.1; or

(d) limit the ability of the Investor and/or any of its Permitted Transferees to exercise such Person's rights under Section 5.2.

#### 4. Restrictions on Dispositions.

**4.1 Lock-up.** During the Lock-up Term, without the prior approval of the Company, the Investor shall not, and shall cause its Affiliates not to, Dispose of (i) any of the Purchased Shares or any Ordinary Shares Beneficially Owned by any Standstill Party as of the date of this Agreement, together with any Ordinary Shares issued in respect thereof as a result of any share split, share dividend, share exchange, merger, consolidation or similar recapitalization, and (ii) any Ordinary Shares issued as (or issuable upon the exercise of any warrant, right or other security that is issued as) a dividend or other distribution with respect to, or in exchange or in replacement of, the Ordinary Shares described in clause (i) of this sentence (collectively, the “**Lock-up Securities**”); provided, however, that the foregoing shall not prohibit the Investor from (x) transferring Lock-up Securities to a Permitted Transferee, (y) transferring Lock-up Securities to a Third Party mutually approved by the Company and the Investor that agrees to be subject to the same restrictions applicable to the Investor hereunder or as provided by the Company, or (z) transferring Lock-up Securities to a Third Party in connection with a business combination transaction approved by the Company.

**4.2 Offering Lock-up.** For so long as the Holders together Beneficially Own at least 4.9% of the Outstanding Ordinary Shares, the Holders shall, if requested by an underwriter of Ordinary Shares of the Company, agree not to Dispose of any Outstanding Ordinary Shares and/or Ordinary Share Equivalents for a specified period of time following the closing of any offering of Ordinary Shares, not to exceed ninety (90) days. The Company may impose stop transfer instructions with respect to the Outstanding Ordinary Shares and/or Ordinary Share Equivalents subject to the foregoing restrictions until the end of the specified period of time. The foregoing provisions of this Section 4.2 shall apply to the Holders only if the Company’s supervisory and management board members and any holders of an equal or greater number of Outstanding Ordinary Shares that are party to a collaboration, license or other similar agreement with the Company are subject to lock-up restrictions that are not less restrictive than those entered into by the Holders. Any discretionary waiver or termination of the restrictions of any or all of such agreements by the underwriters shall apply pro rata to all Holders subject to such agreements, based on the number of shares subject to such agreements.

#### 5. Voting Agreement.

**5.1 Voting of Securities.** Subject to Section 5.2, during the Voting Agreement Term, in any vote of the shareholders of the Company (including, without limitation, with respect to the election of supervisory board members), the Investor undertakes, and shall cause any Permitted Transferees to undertake, subject to applicable Law and Section 9.8, to vote all voting securities of the Company as to which they are entitled to vote, in favor of all items on the agenda for the relevant general meeting of shareholders of the Company as proposed on behalf of the Company.

In furtherance of this Section 5.1, the Investor hereby irrevocably appoints the Company’s supervisory board, and each supervisory director individually, as the attorneys, agents and proxies, with full power of substitution and re-substitution in each of them, for the

Investor, and in the name, place and stead of the Investor, to vote (or cause to be voted) with respect to all voting securities with respect to which the Investor is or may be entitled to vote, in favor of all items on the agenda of any general meeting of shareholders of the Company held after the date hereof as proposed on behalf of the Company, to the extent, but only to the extent set forth in the first paragraph of this Section 5.1, and subject to Section 5.2 (the “**Irrevocable Power of Attorney**”). This Irrevocable Power of Attorney is coupled with an interest, shall be irrevocable and binding on any successor in interest of the Investor and shall not be terminated by operation of law upon the occurrence of any event. This Irrevocable Power of Attorney shall operate to revoke and render void any prior proxy or power of attorney as to voting securities heretofore granted by the Investor which is inconsistent herewith. Notwithstanding the foregoing, the Irrevocable Power of Attorney shall be effective only if, at any annual or extraordinary meeting of the shareholders of the Company (or any consent in lieu thereof) and at any adjournments or postponements of any such meetings, the Investor fails to vote such voting securities in accordance with this Section 5.1 at least two (2) Trading Days prior to the date of such shareholders’ meeting. The Irrevocable Power of Attorney shall terminate upon the earlier of (i) the expiration or termination of the Voting Agreement Term and (ii) the date on which such Irrevocable Power of Attorney is utilized in a manner inconsistent with the terms of the first paragraph of this Section 5.1. The Investor shall cause any Permitted Transferee, if and when requested by the Company from time to time, to promptly execute and deliver to the Company an irrevocable power of attorney, in form and substance satisfactory to the Investor and the Company, and irrevocably appoint the Company’s supervisory board, and each supervisory director individually, with full power of substitution and resubstitution, as its attorney, agent and proxy to vote (or cause to be voted) all of the voting securities of the Company as to which such Permitted Transferee is entitled to vote, in favor of all items on the agenda of any general meeting of shareholders of the Company as proposed on behalf of the Company to the extent, but only to the extent set forth in the first paragraph of this Section 5.1 and subject to Section 5.2 (the “**Permitted Transferee Irrevocable Power of Attorney**”). The Investor acknowledges, and shall cause any Permitted Transferees to acknowledge, that any such proxy executed and delivered shall be coupled with an interest, shall constitute, among other things, an inducement for the Company to enter into this Agreement, shall be irrevocable and binding on any successor in interest of such Permitted Transferee and shall not be terminated by operation of Law upon the occurrence of any event. Such power of attorney shall operate to revoke and render void any prior proxy or power of attorney as to any voting securities of the Company heretofore granted by such Permitted Transferee, to the extent it is inconsistent herewith. The Investor acknowledges and agrees that it shall be a condition to any proposed transfer of voting securities of the Company by the Investor to such Permitted Transferee that such Permitted Transferee execute and deliver to the Company a Permitted Transferee Irrevocable Power of Attorney, and that any purported transfer shall be void and of no force or effect if such Permitted Transferee Irrevocable Power of Attorney is not so executed and delivered at the closing of such transfer. Such power of attorney shall terminate upon the earlier of (i) the expiration or termination of the Voting Agreement Term and (ii) the date on which such Irrevocable Power of Attorney is utilized in a manner inconsistent with the terms of the first paragraph of this Section 5.1. The Investor acknowledges and agrees that it shall be a condition to any proposed transfer of voting securities of the Company by the Investor to any Permitted Transferee that such Permitted Transferee shall agree in writing to be subject to and bound by all restrictions and obligations set forth in this Section 5.1. For purposes of clarification, in no event shall the Irrevocable Power of



the Investor and its Permitted Transferees are permitted to vote their voting securities as desired pursuant to Section 5.2.

**5.2 Certain Transactions Subject to Shareholder Approval.** Notwithstanding the terms of Section 5.1, the Company acknowledges and agrees that the Investor and/or its Permitted Transferees, as applicable, shall have the sole, exclusive and absolute discretion to vote all or any portion of any voting securities of the Company held by such Persons, including any Ordinary Shares held by such Persons, in connection with any vote of the shareholders of the Company made in relation to any transaction of a type set forth in Sections 6.2(a) or 6.2(b) of this Agreement (any such transaction, an “Applicable Control Transaction”, so long as the Investor and/or such Permitted Transferee (or an Affiliate thereof) makes a bona fide, public or private, written offer to the Company, its management board and/or its supervisory board in connection with such Applicable Control Transaction (any such offer, an “Alternative Proposal”) on or prior to the date on which any shareholder vote with respect to such Applicable Control Transaction is held (any such date, the “Initial Shareholder Vote Date”). Furthermore, in the event that (i) the Investor and/or its Permitted Transferees (or an Affiliate thereof) fails to make an Alternative Proposal prior to the Initial Shareholder Vote Date, and (ii) after the Initial Shareholder Vote Date and prior to the consummation of any Applicable Control Transaction, the Company, its management board and/or its supervisory board changes its recommendation to the shareholders of the Company to vote in favor of such Applicable Control Transaction and/or calls and holds another meeting of the shareholders of the Company in order to vote again with respect to such Applicable Control Transaction, then, notwithstanding the failure of the Investor and/or its Permitted Transferees (or an Affiliate thereof) to make an Alternative Proposal prior to the Initial Shareholder Vote Date, and notwithstanding the terms of Section 5.1, the Investor and/or its Permitted Transferees (or an Affiliate thereof) shall be permitted to make an Alternative Proposal with respect to such Applicable Control Transaction, and if such Alternative Proposal is made, the Investor and/or its Permitted Transferees may vote all or any portion of any voting securities of the Company held by such Persons, including any Ordinary Shares held by such Persons, in such Person’s sole, exclusive and absolute discretion with respect to such Applicable Control Transaction. For purposes of clarification, the terms of Sections 5.1 and 5.2 shall (i) in no way alter, amend or otherwise impact the rights of the Investor or any of its Affiliates under Section 3.2(a), which may be made at any time prior to the date on which any Applicable Control Transaction is consummated, or (ii) be applicable in any Applicable Control Transaction in which a vote of the shareholders is not required in order for such transaction to be consummated (e.g., a tender offer).

**6. Termination of Certain Rights and Obligations.**

**6.1 Termination of Registration Rights Term.** Except for Section 2.10, which shall survive until the expiration of any applicable statutes of limitation, Section 2 shall terminate automatically and have no further force or effect upon the earliest to occur of:

- (a) the date on which the Ordinary Shares ceases to be registered pursuant to Section 12 of the Exchange Act; and

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- (b) a liquidation or dissolution of the Company.

**6.2 Termination of Standstill Term.** Section 3 shall terminate and have no further force or effect:

(a) if any Third Party (i) becomes the Beneficial Owner of more than [\*\*] of the Outstanding Ordinary Shares or (ii) commences a tender or exchange offer (which shall be deemed to occur when the offer, offering circular or similar document has been made public) which, if consummated, would make such Third Party (or any of its Affiliates) the Beneficial Owner of more than [\*\*] of the Outstanding Ordinary Shares and the Company does not, within ten (10) Business Days after the commencement of such offer, recommend against shareholders tendering their Ordinary Shares in such offer;

(b) if the Company or any of its Subsidiaries enters into a definitive agreement with any Third Party (A) to divest or sell to such Third Party assets, in one or a series of related transactions (excluding any exclusive or nonexclusive license or joint venture, collaboration or similar transaction), for an aggregate consideration equal to [\*\*] or more of the market capitalization of the Company immediately prior to entering into such transaction, (B) to divest, sell or license to such Third Party, or enter into a joint venture, collaboration or similar transaction with such Third Party involving assets, in one or a series of related transactions, for an aggregate consideration equal to [\*\*] or more of the Outstanding Ordinary Shares immediately following consummation of such transaction, (C) for such Third Party to commence any tender or exchange offer (which shall be deemed to occur when the offer, offering circular or similar document has been made public) that if consummated could result in any Person Beneficially Owning [\*\*] or more of the Outstanding Ordinary Shares or (D) to effectuate a merger, consolidation, business combination, recapitalization or similar transaction that requires the approval of the Company’s shareholders (a “**Merger**”), unless, based on information publicly available at the time of announcement of the entering into of such agreement, immediately following such Merger, (1) no Person would hold more than [\*\*] of the voting power of the corporation resulting from such Merger (the “**Surviving Corporation**”) and (2) more than [\*\*] of the voting equity securities of either (x) the Surviving Corporation, or (y) if applicable, the ultimate parent corporation that directly or indirectly has Beneficial Ownership of all of the outstanding voting equity securities of the Surviving Corporation will be represented by Outstanding Ordinary Shares that were outstanding immediately prior to such Merger or by voting equity securities into which the Ordinary Shares were converted pursuant to such Merger; or

(c) upon the later to occur of (i) the [\*\*] anniversary of the Initial Closing Date; provided, however, that in the event the research term of the Collaboration Agreement is extended pursuant to its terms, then such timeframe shall be automatically extended to the [\*\*] anniversary of the Initial Closing Date; (ii) the [\*\*] anniversary after exercise of the Seventh Target Warrant Agreement; and (iii) the [\*\*] anniversary after exercise of the Tenth Target Warrant Agreement; or

(d) if earlier than any of the dates set forth above, the [\*\*] anniversary of the date on which the Investor or its Affiliates together no longer Beneficially Own at least 4.9% of the Outstanding Ordinary Shares.

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**6.3 Termination of Lock-up Term.** Section 4 shall terminate and have no further force or effect (except for Section 4.2, which shall continue to survive pursuant to its terms) upon the later to occur of:

(a) the [\*\*] anniversary of the Initial Closing Date; provided, however, that in the event the research term of the Collaboration Agreement is extended pursuant to its terms, then such timeframe shall be automatically extended to the [\*\*] anniversary of the Initial Closing

Date; and

(b) in respect of each Ordinary Share acquired pursuant to the Subscription Agreement or the Warrant Agreements, the [\*\*] anniversary of the issuance of each such Ordinary Share;

provided, however, that notwithstanding the foregoing, (x) in the event that the Collaboration Agreement is terminated in whole by Purchaser pursuant to Section 13.3 of the Collaboration Agreement, Section 4 of this Agreement shall terminate on the date on which such termination under the Collaboration Agreement occurs, and (y) in the event that the Collaboration Agreement is otherwise terminated pursuant to its terms, Section 4 of this Agreement will terminate on the one (1) year anniversary of such termination.

**6.4 Termination of Voting Agreement Term.** Section 6 shall terminate and have no further force or effect upon the earlier of (i) the date on which the Investor and any Permitted Transferees together no longer Beneficially Own at least 4.9% of the Outstanding Ordinary Shares, (ii) the closing of any of the types of transactions contemplated by Sections 6.2(a) and 6.2(b), and (iii) the date of termination of the Collaboration Agreement if such Agreement is terminated by Purchaser in whole pursuant to Section 13.3 of the Collaboration Agreement.

**6.5 Effect of Termination.** No termination pursuant to any of Sections 6.1, 6.2, 6.3 or 6.4 shall relieve any of the parties (or a Permitted Transferee, if any) for liability for breach of or default under any of their respective obligations or restrictions under any terminated provision of this Agreement, which breach or default arose out of events or circumstances occurring or existing prior to the date of such termination.

**6.6 Breach of Collaboration Agreement.** Notwithstanding anything to the contrary in the Collaboration Agreement, and in addition to, and not in lieu of, any rights of the Company or the Investors pursuant thereto, if the Company has the right to terminate the Collaboration Agreement (in whole or in part) pursuant to Section 13.3 or 13.4 of the Collaboration Agreement, the Company may, by notice to the Investor, suspend the Company's performance under any or all provisions under this Agreement until the breach giving rise to such right to termination is cured (pursuant to the terms of such agreement) and such suspension of performance shall not be deemed a breach of any obligation by the Company under this Agreement.

## **7. Information Rights.**

**7.1 Information Rights.** The Company shall provide the Investor with (i) for each of the first three fiscal quarters of each fiscal year ending after the Initial Closing Date, not

more than 60 days following the end of such quarter, consolidated unaudited financial statements of the Company and its Subsidiaries consisting of an unaudited income statement for such quarter, statement of cash flows for such quarter and balance sheet as of the end of such quarter and, in each case, prepared in accordance with IFRS, (ii) not more than 120 days following the end of a fiscal year ending after the Initial Closing Date, consolidated audited financial statements of the Company and its Subsidiaries consisting of an audited income statement for such fiscal year, statement of cash flows for such fiscal year and balance sheet as of the end of such fiscal year and, in each case, prepared in accordance with IFRS, and (iii) as soon as reasonably practicable after such request is made, such other information as is reasonably requested by the Investor for it and its Affiliates to meet their respective obligations under applicable Law, including their obligations under the Exchange Act, provided that any documents or other information filed with the SEC need not be separately provided by the Company to the Investor. The Company further agrees to provide the Investor with reasonable access to the books, records, personnel and advisors of the Company to the extent such access is reasonably required in order for the Investor and its Affiliates to meet their respective obligations under applicable Law including their obligations under the Exchange Act.

## **8. Tax Matters.**

**8.1 PFIC Reporting.** The Company hereby agrees to reasonably cooperate with the Investor in order to permit the Investor to determine whether the Company is at any time a "passive foreign investment company" (as defined in Section 1297(a) of the Internal Revenue Code of 1986, as amended (the "**Code**")) (a "**PFIC**"). In furtherance of the foregoing, the Company shall, within ninety (90) days after the end of each taxable year, notify the Investor of its good faith belief as to whether the Company or any current or future direct or indirect Subsidiary of the Company was a PFIC for such taxable year. If the Company or any Subsidiary thereof is determined to be a PFIC, the Company (i) shall promptly after the determination thereof notify Investor, (ii) shall timely provide such information to Investor as Investor may reasonably request to enable Investor to complete its U.S. Internal Revenue Service Form 8621 with respect to such entity and (iii) shall use reasonable best efforts, to provide such statements, information and documentation as Investor reasonably believes is necessary for it to make an election to treat such subsidiary as a "qualified electing fund" under Section 1295 of the Code. The Company shall not be liable to the Investor if its statement of belief, or any information provided pursuant to this Section 8.1, is found to be incorrect, unless such statement of belief or information was not reached or provided in good faith by the Company or was the result of the Company's gross negligence.

**8.2 Controlled Foreign Corporation.** If Investor is a "United States shareholder" within the meaning of Section §951(b) of the Code ("**10% U.S. Shareholder**") of the Company at any point during a taxable year, then the Company hereby agrees to reasonably cooperate with the Investor in order to permit the Investor to determine whether the Company is a "controlled foreign corporation" within the meaning of Section 957 of the Code ("**CFC**"). In furtherance of the foregoing, the Company shall, within ninety (90) days after the end of such taxable year, notify the Investor of its good faith belief, based on the information available to the Company, as to whether the Company is or is likely to have become a CFC for such taxable year. In making the annual determination described above, the Company agrees to inquire of any of its shareholders that hold 10% or more of its voting stock and is not a 10% U.S.

Shareholder whether such shareholder has any direct or indirect owners that could be a 10% U.S. Shareholder of the Company, but shall not be responsible for whether any such shareholder replies, or replies accurately, to such inquiry. If the Company is or is likely to have become a CFC, then the Company shall use reasonable best efforts, to provide to Investor all information reasonably requested by Investor so that Investor may timely comply with its filing obligations under the Code, including but not limited to Internal Revenue Service Form 5471. The Company shall not be liable to the Investor if its statement

of belief, or any information provided pursuant to this Section 8.2, is found to be incorrect, unless such statement of belief or information was not reached or provided in good faith by the Company or was the result of gross negligence.

## **9. Miscellaneous.**

**9.1 Governing Law; Submission to Jurisdiction.** This Agreement shall be governed by and construed in accordance with the Laws of the State of New York, without regard to the conflict of laws principles thereof that would require the application of the Law of any other jurisdiction. The parties irrevocably and unconditionally submit to the exclusive jurisdiction of the United States District Court for the Southern District of New York solely and specifically for the purposes of any action or proceeding arising out of or in connection with this Agreement.

**9.2 Waiver of Jury Trial.** To the extent not prohibited by applicable law which cannot be waived, each party hereto hereby waives and covenants that it will not assert (whether as plaintiff, defendant or otherwise) any right to trial by jury in any forum in respect of any issue or action, claim, cause of action or suit (in contract, tort or otherwise), inquiry, proceeding or investigation arising out of or based upon this Agreement or the subject matter hereof or in any way connected with or related or incidental to the transactions contemplated hereby, in each case whether now existing or hereafter arising. Each party hereto acknowledges that it has been informed by the other parties hereto that this Section 9.2 constitutes a material inducement upon which they are relying and will rely in entering into this Agreement. Any party hereto may file an original counterpart or a copy of this Section 9.2 with any court as written evidence of the consent of each such party to the waiver of its right to trial by jury.

**9.3 Waivers, Delays or Omissions.** It is agreed that no delay or omission to exercise any right, power or remedy accruing to any party, upon any breach, default or noncompliance by another party under this Agreement or the other Transaction Agreements, shall impair any such right, power or remedy, nor shall it be construed to be a waiver of any such breach, default or noncompliance, or any acquiescence therein, or of or in any similar breach, default or noncompliance thereafter occurring. It is further agreed that any waiver, permit, consent or approval of any kind or character on any party's part of any breach, default or noncompliance under this Agreement or the other Transaction Agreements or any waiver on such party's part of any provisions or conditions of this Agreement or the other Transaction Agreements must be in writing and shall be effective only to the extent specifically set forth in such writing. All remedies, either under this Agreement, the other Transaction Agreements, by law, or otherwise afforded to any party, shall be cumulative and not alternative. Any waiver effected in accordance with this Section 9.3 shall be binding upon each party hereto, and any waiver not effected in accordance with this Section 9.3 shall be void and of no effect.

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**9.4 Notices.** All notices required or permitted hereunder shall be in writing and shall be deemed effectively given: (a) upon personal delivery to the party to be notified, (b) when sent by confirmed electronic mail, telex or facsimile if sent during normal business hours of the recipient, if not, then on the next Trading Day, (c) five (5) days after having been sent by registered or certified mail, return receipt requested, postage prepaid or (d) one (1) day after deposit with a nationally recognized overnight courier, specifying next day delivery, with written verification of receipt. All communications shall be sent:

For the Company:

uniQure N.V.  
Meibergdreef 61  
Amsterdam 1105 BA  
The Netherlands  
Attention: Matthew Kapusta, Chief Financial Officer  
Email: m.kapusta@unique.com

With a copy to:

Morgan, Lewis & Bockius LLP  
Condor House, 5-10 St. Paul's Churchyard  
London, EC4M 8AL  
United Kingdom  
Attention: Timothy J. Corbett  
Email: tcorbett@morganlewis.com

For the Investor:

Bristol-Myers Squibb Company  
345 Park Avenue  
New York, New York 10154  
Attention: General Counsel  
Fax: (212) 546-9562

With a copy to:

Bristol-Myers Squibb Pharmaceuticals Group  
Route 206 and Province Line Road  
Princeton, NJ 08543-4000  
Attention: P. Joseph Campisi, Jr.  
Email: joseph.campisi@bms.com

With a further copy (which shall not constitute notice to the Investor) to:

Kirkland & Ellis LLP  
601 Lexington Ave.

Attention: Daniel Wolf and Douglas Ryder  
Email: daniel.wolf@kirkland.com; douglas.ryder@kirkland.com

or such other address or electronic mail address as the Company or Investor may designate by ten (10) days advance written notice to the other parties hereto.

**9.5 Entire Agreement.** This Agreement, the Collaboration Agreement and the Subscription Agreement contain the entire agreement among the parties with respect to the subject matter hereof and thereof and supersede all prior and contemporaneous arrangements or understandings, whether written or oral, with respect hereto and thereto.

**9.6 Amendments.** No provision in this Agreement shall be supplemented, deleted or amended except in a writing executed by an authorized representative of each of the parties hereto.

**9.7 Headings; Nouns and Pronouns; Section References.** Headings in this Agreement are for convenience of reference only and shall not be considered in construing this Agreement. Whenever the context may require, any pronouns used herein shall include the corresponding masculine, feminine or neuter forms, and the singular form of names and pronouns shall include the plural and vice-versa. References in this Agreement to a section or subsection shall be deemed to refer to a section or subsection of this Agreement unless otherwise expressly stated. The words “include,” “includes” and “including” will be deemed to be followed by the phrase “without limitation”. The meanings given to terms defined herein will be equally applicable to both the singular and plural forms of such terms. All references to “dollars” or “\$” will be deemed references to the lawful money of the United States of America.

**9.8 Severability.** In the event one or more of the provisions of this Agreement should, for any reason, be held to be invalid, illegal or unenforceable in any respect, such invalidity, illegality or unenforceability shall not affect any other provisions of this Agreement, and this Agreement shall be construed as if such invalid, illegal or unenforceable provision had never been contained herein. Upon such determination that any provision of this Agreement, or the application of any such provision, is invalid, illegal, void or unenforceable, the Company and the Investor shall negotiate in good faith to modify this Agreement so as to effect the original intent of the Company and the Investor as closely as possible to the fullest extent permitted by Law in an acceptable manner to the end that the transactions contemplated hereby and the other Transaction Agreements are fulfilled to the greatest extent possible.

**9.9 Assignment.** Except for an assignment of this Agreement by the Investor to a Permitted Transferee, neither this Agreement nor any rights or duties of a party hereto may be assigned by such party, in whole or in part, without (a) the prior written consent of the Company in the case of any assignment by the Investor; or (b) the prior written consent of the Investor in the case of an assignment by the Company.

**9.10 Successors and Assigns.** This Agreement shall be binding upon and inure to the benefit of the parties hereto and their respective successors and permitted assigns.

**9.11 Counterparts.** This Agreement may be executed in any number of counterparts (including via facsimile, PDF or other electronic signature), each of which shall be deemed an original but which together shall constitute one and the same instrument.

**9.12 Third Party Beneficiaries.** None of the provisions of this Agreement shall be for the benefit of or enforceable by any Third Party other than any Affiliate of the Investor. No Third Party with the exception of any Affiliate of the Investor 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 any party hereto.

**9.13 No Strict Construction.** This Agreement has been prepared jointly and will not be construed against any party. In the event an ambiguity or question of intent or interpretation arises, this Agreement shall be construed as if drafted jointly by the parties hereto, and no presumption or burden of proof shall arise favoring or disfavoring any party hereto by virtue of the authorship of any provisions of this Agreement.

**9.14 Remedies.** The rights, powers and remedies of the parties under this Agreement are cumulative and not exclusive of any other right, power or remedy which such parties may have under any other agreement or Law. No single or partial assertion or exercise of any right, power or remedy of a party hereunder shall preclude any other or further assertion or exercise thereof.

**9.15 Specific Performance.** The Company and the Investor hereby acknowledge and agree that the rights of the parties hereunder are special, unique and of extraordinary character, and that if any party refuses or otherwise fails to act, or to cause its Affiliates to act, in accordance with the provisions of this Agreement, such refusal or failure would result in irreparable injury to the Company or the Investor, as the case may be, the exact amount of which would be difficult to ascertain or estimate and the remedies at law for which would not be reasonable or adequate compensation. Accordingly, if any party refuses or otherwise fails to act, or to cause its Affiliates to act, in accordance with the provisions of this Agreement, then, in addition to any other remedy which may be available to any damaged party at law or in equity, such damaged party will be entitled to seek specific performance and injunctive relief, without posting bond or other security, and without the necessity of proving actual or threatened damages, which remedy such damaged party will be entitled to seek in any court of competent jurisdiction.

**9.16 Effectiveness.** This Agreement shall become effective on the Initial Closing Date.

[Signature Page to Follow]

IN WITNESS WHEREOF, the parties have executed and delivered this Agreement as of the date first above written.

**UNIQUE N.V.**

By: \_\_\_\_\_  
Name:  
Title:

**BRISTOL-MYERS SQUIBB COMPANY**

By: \_\_\_\_\_  
Name:  
Title:

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

EXECUTION VERSION

## SEVENTH COLLABORATION WARRANT AGREEMENT

### UNIQURE N.V.

THIS SEVENTH COLLABORATION WARRANT AGREEMENT (the “**Warrant**”), dated as of April 6, 2015, certifies that, for value received, Bristol-Myers Squibb Company, a Delaware corporation (the “**Holder**”) is entitled, upon the terms and subject to the limitations on exercise and the conditions hereinafter set forth, to subscribe for and acquire from uniQure N.V., a public company with limited liability (*naamloze vennootschap*) incorporated under the laws of the Netherlands (the “**Company**”), the Warrant Shares, at any time beginning on the Initial Exercise Date and ending at the close of business on the Final Exercise Date, unless this Warrant has previously terminated pursuant to Section 5 hereof. The purchase price per Ordinary Share under this Warrant shall be equal to the Exercise Price.

**Section 1. Definitions.** Capitalized terms used and not otherwise defined herein shall have the meanings set forth in that certain Investor Agreement, dated as of the date hereof, by and between the Company and the Holder (the “**Investor Agreement**”). The following terms shall have the means set forth below:

- (a) “**AGM BMS Transaction Authorization**” shall have the meaning set forth in the Subscription Agreement.
- (b) “**AGM General Authorization**” shall have the meaning set forth in the Subscription Agreement.
- (c) “**Collaboration Agreement**” shall mean the Collaboration and License Agreement, dated as of the date hereof, between the Holder and uniQure Biopharma B.V., an Affiliate of the Company.
- (d) “**Exercise Price**” shall mean, subject to adjustment as set forth in Section 3, a price per Warrant Share equal to the greater of:
  - (i) the product of (A) the purchase price of each Ordinary Share acquired on the Initial Closing Date, multiplied by (B) a compounded annual growth rate of [\*\*] (pro rated daily, if applicable). By way of illustration only, if the purchase price in (A) above is \$30.00 and the date of the Notice of Exercise is eighteen (18) months after the Initial Closing Date, the Exercise Price according to this section (i) would be equal to [\*\*] calculated as follows  $(\$30.00 * (1 + [**]) * (1 + ([**] * (182/364))))$ ; and
  - (ii) the product of (A) [\*\*], multiplied by (B) the VWAP for the twenty (20) Trading Days ending on the date that is five (5) Trading Days prior to the date of a Notice of Exercise delivered by the Holder hereunder.
- (e) “**Final Exercise Date**” shall mean the date [\*\*] following the Initial Exercise Date.
- (f) “**Initial Exercise Date**” shall mean the first Trading Day after the date on which the Target Fees/Designation Condition has been satisfied.

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(g) “**Target Fees/Designation Condition**” shall mean the later of (i) the date on which the Company (or its applicable Affiliate) receives from the Holder the Target Designation Fees (as defined in the Collaboration Agreement) associated with the first [\*\*] (as defined in the Collaboration Agreement) and (ii) the date on which the Holder designates the [\*\*] (as defined in the Collaboration Agreement) in accordance with the terms of the Collaboration Agreement.

(h) “**Subscription Agreement**” shall mean that certain Share Subscription Agreement, dated as of the date hereof, by and between the Company and the Holder.

- (i) “**Warrant Shares**” shall mean, subject to Sections 3 and 6(b):
  - (i) in the event that the AGM BMS Transaction Authorization is obtained, such number of Ordinary Shares (rounded down to the nearest whole share) as shall result in the Holder and its Affiliates beneficially owning 14.90% of the number of Ordinary Shares outstanding immediately following the issuance of such Warrant Shares; or
  - (ii) in the event that the AGM BMS Transaction Authorization is not obtained, such number of Ordinary Shares as will, together with the Ordinary Shares acquired at the Initial Closing and Second Closing (each as defined in the Subscription Agreement), equal 3,317,267.

### **Section 2. Exercise.**

(a) **Condition to Exercise.** Exercise of the purchase rights represented by this Warrant is subject to the satisfaction in full of the Target Fees/Designation Condition.

(b) **Exercise of Warrant.** Exercise of the subscription rights represented by this Warrant may be made in whole (but not in part) at any time on or after the Initial Exercise Date by delivery to the Company (or such other office or agency of the Company as it may designate by notice in writing to the registered Holder at the address of the Holder appearing on the books of the Company) of a duly executed facsimile or electronic copy of the Notice of Exercise form annexed hereto. Within three (3) Trading Days following the date of exercise as aforesaid, the Holder shall deliver to the Company the aggregate Exercise Price by wire transfer to an account designated by the Company. In addition, the Holder shall surrender this Warrant to the Company for

cancellation within three (3) Trading Days of the date the Notice of Exercise is delivered to the Company. The Company shall deliver any objection to any Notice of Exercise form within one (1) Business Day of receipt of such notice.

(c) Mechanics of Exercise.

(i) Delivery of Warrant Shares Upon Exercise. The Company shall cause the Warrant Shares purchased hereunder to be delivered to the Holder by book-entry delivery by the date that is five (5) Trading Days after payment of the aggregate Exercise Price in respect thereof (such date, the “**Warrant Share Delivery Date**”). The Warrant Shares shall be deemed to have been issued, and the Holder shall be deemed to have become a holder of record of such Warrant Shares for all purposes, as of the date on which the aggregate Exercise Price has been received.

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(ii) Closing of Books. The Company will not close its shareholder books or records in any manner which prevents the timely exercise of this Warrant, pursuant to the terms hereof.

Section 3. Share Dividends and Splits. If the Company, at any time while this Warrant is outstanding: (i) pays a share dividend or otherwise makes a distribution or distributions on its Ordinary Shares or any other equity or equity equivalent securities payable in Ordinary Shares (which, for avoidance of doubt, shall not include any Ordinary Shares issued by the Company upon exercise of this Warrant), (ii) subdivides outstanding Ordinary Shares into a larger number of shares, (iii) combines (including by way of reverse share split) outstanding Ordinary Shares into a smaller number of shares or (iv) issues by reclassification of shares of the Ordinary Shares any shares in the capital of the Company, then in each case the Exercise Price shall (for purposes of Section 1(d)(i)) be multiplied by a fraction of which the numerator shall be the number of Ordinary Shares (excluding treasury shares, if any) outstanding immediately before such event and of which the denominator shall be the number of Ordinary Shares outstanding immediately after such event, and the number of Warrant Shares issuable upon exercise of this Warrant shall be proportionately adjusted such that the aggregate Exercise Price calculated pursuant to Section 1(d)(i) shall remain unchanged, and the percentage of the total number of Ordinary Shares outstanding (after giving effect to such event) represented by the Warrant Shares shall remain unchanged. Any adjustment made pursuant to this Section 3 shall become effective immediately after the record date for the determination of shareholders entitled to receive such dividend or distribution and shall become effective immediately after the effective date in the case of a subdivision, combination or reclassification.

Section 4. Transfer of Warrant. This Warrant is not transferable by the Holder; provided, that the Holder may transfer this Warrant to a Permitted Transferee.

Section 5. Termination; Suspension of Exercisability.

(a) If not previously exercised pursuant to the terms hereof, this Warrant shall terminate and expire upon the earlier of (i) the Final Exercise Date and (ii) the termination of the Collaboration Agreement.

(b) Notwithstanding the foregoing, in the event that BMS (as defined in the Collaboration Agreement) ceases to use Diligent Efforts (as defined in the Collaboration Agreement) to Develop (as defined in the Collaboration Agreement) at least [\*\*] Research Programs (as defined in the Collaboration Agreement), the Company may, by notice to the Holder, suspend the Company’s performance under any or all provisions under this Warrant until such failure is cured (pursuant to the terms of the Collaboration Agreement), and such suspension of performance shall not be deemed a breach of any obligation by the Company under this Warrant. Any determination as to whether BMS has ceased to use Diligent Efforts in respect of at least [\*\*] Research Programs shall be made, and any dispute in respect thereof shall be resolved in accordance with, the provisions of Section 13.4 of the Collaboration Agreement.

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Section 6. Miscellaneous.

(a) No Rights as Shareholder until Exercise. This Warrant does not entitle the Holder to any voting rights, profit rights or other rights as a shareholder of the Company in respect of the Warrant Shares prior to the delivery of the Warrant Shares pursuant to the terms hereof.

(b) Merger or Reorganization. If at any time there shall be any reorganization, recapitalization, merger or consolidation (a “**Reorganization**”) involving the Company in which the Ordinary Shares are converted into or exchanged for securities, cash or other property, then, as a part of such Reorganization, lawful provision shall be made so that the Holder shall thereafter be entitled to receive upon exercise of this Warrant, the kind and amount of securities, cash or other property of the successor entity resulting from such Reorganization, equivalent in value to that which a holder of Ordinary Shares would have been entitled in such Reorganization if the right to acquire the Ordinary Shares hereunder had been exercised immediately prior to such Reorganization. In any such case, appropriate adjustment shall be made in the application of the provisions of this Warrant with respect to the rights and interests of the Holder after such Reorganization to the end that the provisions of this Warrant shall be applicable after the event, as near as practically may be, in relation to any securities deliverable after that event upon the exercise of this Warrant.

(c) Loss, Theft, Destruction or Mutilation of Warrant. The Company covenants that upon receipt by the Company of evidence reasonably satisfactory to it of the loss, theft, destruction or mutilation of this Warrant, and in case of loss, theft or destruction, of indemnity or security reasonably satisfactory to it (which, in the case of the Warrant, shall not include the posting of any bond), and upon surrender and cancellation of such Warrant, if mutilated, the Company will make and deliver a new Warrant of like tenor and dated as of such cancellation, in lieu of such Warrant.

(d) Saturdays, Sundays, Holidays, etc. If the last or appointed day for the taking of any action or the expiration of any right required or granted herein shall not be a Trading Day, then, such action may be taken or such right may be exercised on the next succeeding Trading Day.

(e) Authorized Shares. The Company covenants that, upon receipt of the AGM BMS Transaction Authorization or the AGM General Authorization and during the period the Warrant is outstanding, it will reserve from its authorized capital (*maatschappelijke kapitaal*) a sufficient amount to provide for the issuance of the Warrant Shares upon the exercise of any acquire rights under this Warrant. The Company will assure that such Warrant Shares may be issued as provided herein without violation of any applicable Law or regulation, or of any requirements of the Trading Market upon which the Ordinary Shares may be listed. The Company covenants that all Warrant Shares which may be issued upon the exercise of the subscription rights represented

by this Warrant will, upon exercise of the subscription rights represented by this Warrant and payment of the Exercise Price in respect thereof, be duly authorized, validly issued, fully paid and free from all taxes, liens and charges created by the Company in respect of the issue thereof (other than taxes in respect of any transfer occurring contemporaneously with such issue).

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(f) **Jurisdiction.** This Warrant shall be governed by and construed in accordance with the Laws of the State of New York, without regard to the conflict of laws principles thereof that would require the application of the Law of any other jurisdiction. The parties irrevocably and unconditionally submit to the exclusive jurisdiction of the United States District Court for the Southern District of New York solely and specifically for the purposes of any action or proceeding arising out of or in connection with this Warrant.

(g) **Waiver of Jury Trial.** To the extent not prohibited by applicable Law that cannot be waived, each of the Company and the Holder hereby waives and covenants that it will not assert (whether as plaintiff, defendant or otherwise) any right to trial by jury in any forum in respect of any issue or action, claim, cause of action or suit (in contract, tort or otherwise), inquiry, proceeding or investigation arising out of or based upon this Warrant or the subject matter hereof or in any way connected with or related or incidental to the transactions contemplated hereby, in each case whether now existing or hereafter arising. Each of the Company and the Holder acknowledge that it has been informed by the other that this Section 6(g) constitutes a material inducement upon which they are relying and will rely in entering into and exercising this Warrant. Either of the Company or the Holder may file an original counterpart or a copy of this Section 6(g) with any court as written evidence of the consent of each such party to the waiver of its right to trial by jury.

(h) **Restrictions.** The Holder acknowledges that the Warrant Shares acquired upon the exercise of this Warrant, if not registered, will have restrictions upon resale imposed by all applicable securities laws.

(i) **Waivers; Delays or Omissions.** It is agreed that no delay or omission to exercise any right, power or remedy accruing to any party, upon any breach, default or noncompliance by another party under this Warrant shall impair any such right, power or remedy, nor shall it be construed to be a waiver of any such breach, default or noncompliance, or any acquiescence therein, or of or in any similar breach, default or noncompliance thereafter occurring. It is further agreed that any consent of any kind or character on any party's part of any breach, default or noncompliance under this Warrant or any waiver on such party's part of any provisions or conditions of the Warrant must be in writing and shall be effective only to the extent specifically set forth in such writing. All remedies, either under this Warrant by law, or otherwise afforded to any party, shall be cumulative and not alternative. Any waiver effected in accordance with this Section 6(i) shall be binding upon the Company and the Holder, and any waiver not effected in accordance with this Section 6(i) shall be void and of no effect.

(j) **Notices.** Any notice, request or other document required or permitted to be given or delivered to the Holder by the Company shall be delivered in accordance with the notice provisions of the Investor Agreement.

(k) **Limitation of Liability.** No provision hereof, in the absence of any affirmative action by the Holder to exercise this Warrant to acquire Warrant Shares, and no enumeration herein of the rights or privileges of the Holder, shall give rise to any liability of the Holder for the purchase price of any Ordinary Shares or as a shareholder of the Company, whether such liability is asserted by the Company or by creditors of the Company.

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(l) **Successors and Assigns.** Subject to applicable securities laws, this Warrant and the rights and obligations evidenced hereby shall inure to the benefit of and be binding upon the successors and permitted assigns of the Company and the successors and permitted assigns of the Holder.

(m) **Amendment.** No provision in this Warrant shall be supplemented, deleted or amended except in a writing executed by an authorized representative of each of the Holder and the Company, and any amendment not effected in accordance with this Section 6(m) shall be void and of no effect.

(n) **Severability.** In the event one or more of the provisions of this Warrant should, for any reason, be held to be invalid, illegal or unenforceable in any respect, such invalidity, illegality or unenforceability shall not affect any other provisions of this Warrant, and this Warrant shall be construed as if such invalid, illegal or unenforceable provision had never been contained herein. Upon such determination that any provision of this Warrant, or the application of any such provision, is invalid, illegal, void or unenforceable, the Company and the Holder shall negotiate in good faith to modify this Warrant so as to effect the original intent of the Company and the original Holder as closely as possible to the fullest extent permitted by law in an acceptable manner to the end that the transactions contemplated hereby are fulfilled to the greatest extent possible.

(o) **Titles and Subtitles.** The titles of the sections and subsections of this Warrant are for convenience of reference only and are not to be considered in construing this Warrant.

[Signature Page to Follow]

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IN WITNESS WHEREOF, the Company has caused this Warrant to be executed by its officer thereunto duly authorized as of the date first above indicated.

**UNIQURE N.V.**

By: \_\_\_\_\_  
Name: \_\_\_\_\_



Title:

[Signature Page to the Seventh Target Warrant Agreement]

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**NOTICE OF EXERCISE**

TO:     UNIQUE N.V.

(1)       The undersigned hereby elects to acquire the Warrant Shares pursuant to the terms of the attached Warrant and tenders herewith payment of the exercise price in full.

(2)       Please issue said Warrant Shares in the name of the undersigned or in such other name as is specified below:

[SIGNATURE OF HOLDER]

Name of Investing Entity: \_\_\_\_\_

*Signature of Authorized Signatory of Investing Entity:* \_\_\_\_\_

Name of Authorized Signatory: \_\_\_\_\_

Title of Authorized Signatory: \_\_\_\_\_

Date: \_\_\_\_\_

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

EXECUTION VERSION

## TENTH COLLABORATION WARRANT AGREEMENT

### UNIQURE N.V.

THIS TENTH COLLABORATION WARRANT AGREEMENT (the “**Warrant**”), dated as of April 6, 2015, certifies that, for value received, Bristol-Myers Squibb Company, a Delaware corporation (the “**Holder**”) is entitled, upon the terms and subject to the limitations on exercise and the conditions hereinafter set forth, to subscribe for and acquire from uniQure N.V., a public company with limited liability (*naamloze vennootschap*) incorporated under the laws of the Netherlands (the “**Company**”), the Warrant Shares, at any time beginning on the Initial Exercise Date and ending at the close of business on the Final Exercise Date, unless this Warrant has previously terminated pursuant to Section 5 hereof. The purchase price per Ordinary Share under this Warrant shall be equal to the Exercise Price.

**Section 1. Definitions.** Capitalized terms used and not otherwise defined herein shall have the meanings set forth in that certain Investor Agreement, dated as of the date hereof, by and between the Company and the Holder (the “**Investor Agreement**”). The following terms shall have the means set forth below:

- (a) “**AGM BMS Transaction Authorization**” shall have the meaning set forth in the Subscription Agreement.
- (b) “**AGM General Authorization**” shall have the meaning set forth in the Subscription Agreement.
- (c) “**Collaboration Agreement**” shall mean the Collaboration and License Agreement, dated as of the date hereof, between the Holder and uniQure Biopharma B.V., an Affiliate of the Company.
- (d) “**Exercise Price**” shall mean, subject to adjustment as set forth in Section 3, a price per Warrant Share equal to the greater of:
  - (i) the product of (A) the purchase price of each Ordinary Share acquired on the Initial Closing Date, multiplied by (B) a compounded annual growth rate of [\*\*] (pro rated daily, if applicable). By way of illustration only, if the purchase price in (A) above is \$30.00 and the date of the Notice of Exercise is eighteen (18) months after the Initial Closing Date, the Exercise Price according to this section (i) would be equal to [\*\*] calculated as follows  $(\$30.00 * (1 + [**]) * (1 + ([**] * (182/364))))$ ; and
  - (ii) the product of (A) [\*\*], multiplied by (B) the VWAP for the twenty (20) Trading Days ending on the date that is five (5) Trading Days prior to the date of a Notice of Exercise delivered by the Holder hereunder.
- (e) “**Final Exercise Date**” shall mean the date [\*\*] following the Initial Exercise Date.
- (f) “**Initial Exercise Date**” shall mean the first Trading Day after the date on which the Target Fees/Designation Condition has been satisfied.

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(g) “**Target Fees/Designation Condition**” shall mean the later of (i) the date on which the Company (or its applicable Affiliate) receives from the Holder the Target Designation Fees (as defined in the Collaboration Agreement) associated with each of the first [\*\*] (as defined in the Collaboration Agreement) and (ii) the date on which the Holder designates the [\*\*] New Target (as defined in the Collaboration Agreement) in accordance with the terms of the Collaboration Agreement.

(h) “**Subscription Agreement**” shall mean that certain Share Subscription Agreement, dated as of the date hereof, by and between the Company and the Holder.

- (i) “**Warrant Shares**” shall mean, subject to Sections 3 and 6(b):
  - (i) in the event that the AGM BMS Transaction Authorization is obtained, such number of Ordinary Shares (rounded down to the nearest whole share) as shall result in the Holder and its Affiliates beneficially owning 19.90% of the number of Ordinary Shares outstanding immediately following the issuance of such Warrant Shares; or
  - (ii) in the event that the AGM BMS Transaction Authorization is not obtained, such number of Ordinary Shares as will, together with the Ordinary Shares acquired at the Initial Closing and Second Closing (each as defined in the Subscription Agreement) and pursuant to the exercise of the Seventh Target Warrant Agreement, equal 3,667,423.

### **Section 2. Exercise.**

(a) **Condition to Exercise.** Exercise of the purchase rights represented by this Warrant is subject to the satisfaction in full of the Target Fees/Designation Condition.

(b) **Exercise of Warrant.** Exercise of the subscription rights represented by this Warrant may be made in whole (but not in part) at any time on or after the Initial Exercise Date by delivery to the Company (or such other office or agency of the Company as it may designate by notice in writing to the registered Holder at the address of the Holder appearing on the books of the Company) of a duly executed facsimile or electronic copy of the Notice of Exercise form annexed hereto. Within three (3) Trading Days following the date of exercise as aforesaid, the Holder shall deliver to the Company the aggregate Exercise Price by wire transfer to an account designated by the Company. In addition, the Holder shall surrender this Warrant to the Company for

cancellation within three (3) Trading Days of the date the Notice of Exercise is delivered to the Company. The Company shall deliver any objection to any Notice of Exercise form within one (1) Business Day of receipt of such notice.

(c) Mechanics of Exercise.

(i) Delivery of Warrant Shares Upon Exercise. The Company shall cause the Warrant Shares purchased hereunder to be delivered to the Holder by book-entry delivery by the date that is five (5) Trading Days after payment of the aggregate Exercise Price in respect thereof (such date, the “**Warrant Share Delivery Date**”). The Warrant Shares shall be deemed to have

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been issued, and the Holder shall be deemed to have become a holder of record of such Warrant Shares for all purposes, as of the date on which the aggregate Exercise Price has been received.

(ii) Closing of Books. The Company will not close its shareholder books or records in any manner which prevents the timely exercise of this Warrant, pursuant to the terms hereof.

Section 3. Share Dividends and Splits. If the Company, at any time while this Warrant is outstanding: (i) pays a share dividend or otherwise makes a distribution or distributions on its Ordinary Shares or any other equity or equity equivalent securities payable in Ordinary Shares (which, for avoidance of doubt, shall not include any Ordinary Shares issued by the Company upon exercise of this Warrant), (ii) subdivides outstanding Ordinary Shares into a larger number of shares, (iii) combines (including by way of reverse share split) outstanding Ordinary Shares into a smaller number of shares or (iv) issues by reclassification of shares of the Ordinary Shares any shares in the capital of the Company, then in each case the Exercise Price shall (for purposes of Section 1(d)(i)) be multiplied by a fraction of which the numerator shall be the number of Ordinary Shares (excluding treasury shares, if any) outstanding immediately before such event and of which the denominator shall be the number of Ordinary Shares outstanding immediately after such event, and the number of Warrant Shares issuable upon exercise of this Warrant shall be proportionately adjusted such that the aggregate Exercise Price calculated pursuant to Section 1(d)(i) shall remain unchanged, and the percentage of the total number of Ordinary Shares outstanding (after giving effect to such event) represented by the Warrant Shares shall remain unchanged. Any adjustment made pursuant to this Section 3 shall become effective immediately after the record date for the determination of shareholders entitled to receive such dividend or distribution and shall become effective immediately after the effective date in the case of a subdivision, combination or reclassification.

Section 4. Transfer of Warrant. This Warrant is not transferable by the Holder; provided, that the Holder may transfer this Warrant to a Permitted Transferee.

Section 5. Termination; Suspension of Exercisability.

(a) If not previously exercised pursuant to the terms hereof, this Warrant shall terminate and expire upon the earlier of (i) the Final Exercise Date and (ii) the termination of the Collaboration Agreement.

(b) Notwithstanding the foregoing, in the event that BMS (as defined in the Collaboration Agreement) ceases to use Diligent Efforts (as defined in the Collaboration Agreement) to Develop (as defined in the Collaboration Agreement) at least [\*\*] Research Programs (as defined in the Collaboration Agreement), the Company may, by notice to the Holder, suspend the Company’s performance under any or all provisions under this Warrant until such failure is cured (pursuant to the terms of the Collaboration Agreement), and such suspension of performance shall not be deemed a breach of any obligation by the Company under this Warrant. Any determination as to whether BMS has ceased to use Diligent Efforts in respect of at least [\*\*] Research Programs shall be made, and any dispute in respect thereof shall be resolved in accordance with, the provisions of Section 13.4 of the Collaboration Agreement.

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Section 6. Miscellaneous.

(a) No Rights as Shareholder until Exercise. This Warrant does not entitle the Holder to any voting rights, profit rights or other rights as a shareholder of the Company in respect of the Warrant Shares prior to the delivery of the Warrant Shares pursuant to the terms hereof.

(b) Merger or Reorganization. If at any time there shall be any reorganization, recapitalization, merger or consolidation (a “**Reorganization**”) involving the Company in which the Ordinary Shares are converted into or exchanged for securities, cash or other property, then, as a part of such Reorganization, lawful provision shall be made so that the Holder shall thereafter be entitled to receive upon exercise of this Warrant, the kind and amount of securities, cash or other property of the successor entity resulting from such Reorganization, equivalent in value to that which a holder of Ordinary Shares would have been entitled in such Reorganization if the right to acquire the Ordinary Shares hereunder had been exercised immediately prior to such Reorganization. In any such case, appropriate adjustment shall be made in the application of the provisions of this Warrant with respect to the rights and interests of the Holder after such Reorganization to the end that the provisions of this Warrant shall be applicable after the event, as near as practically may be, in relation to any securities deliverable after that event upon the exercise of this Warrant.

(c) Loss, Theft, Destruction or Mutilation of Warrant. The Company covenants that upon receipt by the Company of evidence reasonably satisfactory to it of the loss, theft, destruction or mutilation of this Warrant, and in case of loss, theft or destruction, of indemnity or security reasonably satisfactory to it (which, in the case of the Warrant, shall not include the posting of any bond), and upon surrender and cancellation of such Warrant, if mutilated, the Company will make and deliver a new Warrant of like tenor and dated as of such cancellation, in lieu of such Warrant.

(d) Saturdays, Sundays, Holidays, etc. If the last or appointed day for the taking of any action or the expiration of any right required or granted herein shall not be a Trading Day, then, such action may be taken or such right may be exercised on the next succeeding Trading Day.

(e) Authorized Shares. The Company covenants that, upon receipt of the AGM BMS Transaction Authorization or the AGM General Authorization and during the period the Warrant is outstanding, it will reserve from its authorized capital (*maatschappelijke kapitaal*) a sufficient amount to provide for the issuance of the Warrant Shares upon the exercise of any acquire rights under this Warrant. The Company will assure that such Warrant Shares

may be issued as provided herein without violation of any applicable Law or regulation, or of any requirements of the Trading Market upon which the Ordinary Shares may be listed. The Company covenants that all Warrant Shares which may be issued upon the exercise of the subscription rights represented by this Warrant will, upon exercise of the subscription rights represented by this Warrant and payment of the Exercise Price in respect thereof, be duly authorized, validly issued, fully paid and free from all taxes, liens and charges created by the Company in respect of the issue thereof (other than taxes in respect of any transfer occurring contemporaneously with such issue).

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(f) **Jurisdiction.** This Warrant shall be governed by and construed in accordance with the Laws of the State of New York, without regard to the conflict of laws principles thereof that would require the application of the Law of any other jurisdiction. The parties irrevocably and unconditionally submit to the exclusive jurisdiction of the United States District Court for the Southern District of New York solely and specifically for the purposes of any action or proceeding arising out of or in connection with this Warrant.

(g) **Waiver of Jury Trial.** To the extent not prohibited by applicable Law that cannot be waived, each of the Company and the Holder hereby waives and covenants that it will not assert (whether as plaintiff, defendant or otherwise) any right to trial by jury in any forum in respect of any issue or action, claim, cause of action or suit (in contract, tort or otherwise), inquiry, proceeding or investigation arising out of or based upon this Warrant or the subject matter hereof or in any way connected with or related or incidental to the transactions contemplated hereby, in each case whether now existing or hereafter arising. Each of the Company and the Holder acknowledge that it has been informed by the other that this Section 6(g) constitutes a material inducement upon which they are relying and will rely in entering into and exercising this Warrant. Either of the Company or the Holder may file an original counterpart or a copy of this Section 6(g) with any court as written evidence of the consent of each such party to the waiver of its right to trial by jury.

(h) **Restrictions.** The Holder acknowledges that the Warrant Shares acquired upon the exercise of this Warrant, if not registered, will have restrictions upon resale imposed by all applicable securities laws.

(i) **Waivers; Delays or Omissions.** It is agreed that no delay or omission to exercise any right, power or remedy accruing to any party, upon any breach, default or noncompliance by another party under this Warrant shall impair any such right, power or remedy, nor shall it be construed to be a waiver of any such breach, default or noncompliance, or any acquiescence therein, or of or in any similar breach, default or noncompliance thereafter occurring. It is further agreed that any consent of any kind or character on any party's part of any breach, default or noncompliance under this Warrant or any waiver on such party's part of any provisions or conditions of the Warrant must be in writing and shall be effective only to the extent specifically set forth in such writing. All remedies, either under this Warrant by law, or otherwise afforded to any party, shall be cumulative and not alternative. Any waiver effected in accordance with this Section 6(i) shall be binding upon the Company and the Holder, and any waiver not effected in accordance with this Section 6(i) shall be void and of no effect.

(j) **Notices.** Any notice, request or other document required or permitted to be given or delivered to the Holder by the Company shall be delivered in accordance with the notice provisions of the Investor Agreement.

(k) **Limitation of Liability.** No provision hereof, in the absence of any affirmative action by the Holder to exercise this Warrant to acquire Warrant Shares, and no enumeration herein of the rights or privileges of the Holder, shall give rise to any liability of the Holder for the purchase price of any Ordinary Shares or as a shareholder of the Company, whether such liability is asserted by the Company or by creditors of the Company.

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(l) **Successors and Assigns.** Subject to applicable securities laws, this Warrant and the rights and obligations evidenced hereby shall inure to the benefit of and be binding upon the successors and permitted assigns of the Company and the successors and permitted assigns of the Holder.

(m) **Amendment.** No provision in this Warrant shall be supplemented, deleted or amended except in a writing executed by an authorized representative of each of the Holder and the Company, and any amendment not effected in accordance with this Section 6(m) shall be void and of no effect.

(n) **Severability.** In the event one or more of the provisions of this Warrant should, for any reason, be held to be invalid, illegal or unenforceable in any respect, such invalidity, illegality or unenforceability shall not affect any other provisions of this Warrant, and this Warrant shall be construed as if such invalid, illegal or unenforceable provision had never been contained herein. Upon such determination that any provision of this Warrant, or the application of any such provision, is invalid, illegal, void or unenforceable, the Company and the Holder shall negotiate in good faith to modify this Warrant so as to effect the original intent of the Company and the original Holder as closely as possible to the fullest extent permitted by law in an acceptable manner to the end that the transactions contemplated hereby are fulfilled to the greatest extent possible.

(o) **Titles and Subtitles.** The titles of the sections and subsections of this Warrant are for convenience of reference only and are not to be considered in construing this Warrant.

[Signature Page to Follow]

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IN WITNESS WHEREOF, the Company has caused this Warrant to be executed by its officer thereunto duly authorized as of the date first above indicated.

UNIQUE N.V.

By: \_\_\_\_\_  
Name: \_\_\_\_\_  
Title: \_\_\_\_\_

[Signature Page to the Tenth Target Warrant Agreement]

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**NOTICE OF EXERCISE**

TO:     UNIQUE N.V.

(1)       The undersigned hereby elects to acquire the Warrant Shares pursuant to the terms of the attached Warrant and tenders herewith payment of the exercise price in full.

(2)       Please issue said Warrant Shares in the name of the undersigned or in such other name as is specified below:

[SIGNATURE OF HOLDER]

Name of Investing Entity: \_\_\_\_\_

*Signature of Authorized Signatory of Investing Entity:* \_\_\_\_\_

Name of Authorized Signatory: \_\_\_\_\_

Title of Authorized Signatory: \_\_\_\_\_

Date: \_\_\_\_\_

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## SUBSIDIARIES OF UNIQUE N.V.

Name of Subsidiary	Jurisdiction of Organization
uniQure biopharma B.V.	The Netherlands
uniQure IP B.V.	The Netherlands
uniQure Manufacturing B.V.	The Netherlands
uniQure Assay Development B.V.	The Netherlands
uniQure Research B.V.	The Netherlands
uniQure non clinical B.V.	The Netherlands
uniQure QA B.V.	The Netherlands
uniQure Process Development B.V.	The Netherlands
uniQure clinical B.V.	The Netherlands
uniQure Inc.	Delaware
uniQure GmbH	Germany

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**Certification by the Chief Executive Officer  
Pursuant to Section 302 of the Sarbanes-Oxley Act of 2002**

I, Jörn Aldag, certify that:

1. I have reviewed this annual report on Form 20-F of uniQure N.V. (the “Company”);
2. Based on my knowledge, this report does not contain any untrue statement of a material fact or omit to state a material fact necessary to make the statements made, in light of the circumstances under which such statements were made, not misleading with respect to the period covered by this report;
3. Based on my knowledge, the financial statements, and other financial information included in this report, fairly present in all material respects the financial condition, results of operations and cash flows of the Company as of, and for, the periods presented in this report;
4. The Company’s other certifying officer and I are responsible for establishing and maintaining disclosure controls and procedures (as defined in Exchange Act Rules 13a-15(e) and 15d-15(e)) and internal control over financial reporting (as defined in Exchange Act Rules 13a 15(f) and 15d 15(f)) for the Company and have:
  - a. Designed such disclosure controls and procedures, or caused such disclosure controls and procedures to be designed under our supervision, to ensure that material information relating to the Company, including its consolidated subsidiaries, is made known to us by others within those entities, particularly during the period in which this report is being prepared;
  - b. Designed such internal control over financial reporting, or caused such internal control over financial reporting to be designed under our supervision, to provide reasonable assurance regarding the reliability of financial reporting and the preparation of financial statements for external purposes in accordance with generally accepted accounting principles;
  - c. Evaluated the effectiveness of the Company’s disclosure controls and procedures and presented in this report our conclusions about the effectiveness of the disclosure controls and procedures, as of the end of the period covered by this report based on such evaluation;
  - d. Disclosed in this report any change in the Company’s internal control over financial reporting that occurred during the period covered by the annual report that has materially affected, or is reasonably likely to materially affect, the Company’s internal control over financial reporting; and
5. The Company’s other certifying officer and I have disclosed, based on our most recent evaluation of internal control over financial reporting, to the Company’s auditors and the audit committee of the Company’s board of directors (or persons performing the equivalent functions):
  - a. All significant deficiencies and material weaknesses in the design or operation of internal control over financial reporting which are reasonably likely to adversely affect the Company’s ability to record, process, summarize and report financial information; and
  - b. Any fraud, whether or not material, that involves management or other employees who have a significant role in the Company’s internal control over financial reporting.

Date: April 7, 2015

By: /s/ JÖRN ALDAG

Name: Jörn Aldag

Title: *Chief Executive Officer*

**Certification by the Chief Financial Officer  
Pursuant to Section 302 of the Sarbanes-Oxley Act of 2002**

I, Matthew Kapusta, certify that:

1. I have reviewed this annual report on Form 20-F of uniQure N.V. (the "Company");
2. Based on my knowledge, this report does not contain any untrue statement of a material fact or omit to state a material fact necessary to make the statements made, in light of the circumstances under which such statements were made, not misleading with respect to the period covered by this report;
3. Based on my knowledge, the financial statements, and other financial information included in this report, fairly present in all material respects the financial condition, results of operations and cash flows of the Company as of, and for, the periods presented in this report;
4. The Company's other certifying officer and I are responsible for establishing and maintaining disclosure controls and procedures (as defined in Exchange Act Rules 13a-15(e) and 15d-15(e)) and internal control over financial reporting (as defined in Exchange Act Rules 13a-15(f) and 15d-15(f)) for the Company and have:
  - a. Designed such disclosure controls and procedures, or caused such disclosure controls and procedures to be designed under our supervision, to ensure that material information relating to the Company, including its consolidated subsidiaries, is made known to us by others within those entities, particularly during the period in which this report is being prepared;
  - b. Evaluated the effectiveness of the Company's disclosure controls and procedures and presented in this report our conclusions about the effectiveness of the disclosure controls and procedures, as of the end of the period covered by this report based on such evaluation; and
  - c. Disclosed in this report any change in the Company's internal control over financial reporting that occurred during the period covered by the annual report that has materially affected, or is reasonably likely to materially affect, the Company's internal control over financial reporting.
5. The Company's other certifying officer and I have disclosed, based on our most recent evaluation of internal control over financial reporting, to the Company's auditors and the audit committee of the Company's board of directors (or persons performing the equivalent functions):
  - a. All significant deficiencies and material weaknesses in the design or operation of internal control over financial reporting which are reasonably likely to adversely affect the Company's ability to record, process, summarize and report financial information; and
  - b. Any fraud, whether or not material, that involves management or other employees who have a significant role in the Company's internal control over financial reporting.

Date: April 7, 2015

By: /s/ MATTHEW KAPUSTA

Name: Matthew Kapusta

Title: *Chief Financial Officer*



**Certification by the Chief Executive Officer and Chief Financial Officer pursuant to 18 U.S.C.  
Section 1350, as adopted pursuant to Section 906 of the Sarbanes-Oxley Act of 2002**

In connection with the Annual Report on Form 20-F of uniQure N.V. (the “Company”) for the year ended December 31, 2014, as filed with the U.S. Securities and Exchange Commission on the date hereof (the “Report”), the undersigned Jörn Aldag, as Chief Executive Officer of the Company, and Matthew Kapusta, as Chief Financial Officer of the Company, each hereby certifies, pursuant to 18 U.S.C. Section 1350, as adopted pursuant to Section 906 of the Sarbanes-Oxley Act of 2002, that to the best of his knowledge:

1. The Report fully complies with the requirements of Section 13(a) or 15(d) of the Securities Exchange Act of 1934; and
2. The information contained in the Report fairly presents, in all material respects, the financial condition and results of operations of the Company.

Date: April 7, 2015

By: /s/ JÖRN ALDAG

Name: Jörn Aldag

Title: *Chief Executive Officer*

By: /s/ MATTHEW KAPUSTA

Name: Matthew Kapusta

Title: *Chief Financial Officer*

CONSENT OF INDEPENDENT REGISTERED PUBLIC ACCOUNTING FIRM

We hereby consent to the incorporation by reference in the Registration Statements on Form S-8 (No. 333-197887) and on Form F-3 (No. 333-202456) of uniQure N.V. of our report dated April 7, 2015 relating to the financial statements of uniQure N.V., which appears in this Form 20-F.

/s/ PricewaterhouseCoopers Accountants N.V.

drs. A.C.M. van der Linden RA  
Utrecht, Netherlands  
April 7, 2015

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