Use these links to rapidly review the document
TABLE OF CONTENTS
UNIQURE N.V. INDEX TO CONSOLIDATED FINANCIAL STATEMENTS

Table of Contents

SECURITIES AND EXCHANGE COMMISSION

Washington D.C. 20549

FORM 20-F

o **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, 2013

OR

o **TRANSITION REPORT PURSUANT TO SECTION 13 OR 15(d) OF THE SECURITIES EXCHANGE ACT OF 1934**
For the transition period from to

o **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

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 20 566 7394
Fax: +31 20 566 9272
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:

Title of each class	Name of each exchange on which registered
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:

12,194,906 Ordinary Shares
(as of December 31, 2013)

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

o 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.

o 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 o 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 o 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 o	Accelerated filer o	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 o	International Financial Reporting Standards as issued by the International Accounting Standards Board ☒	Other o

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:

o Item 17 o 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).

o 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.

o Yes o No

TABLE OF CONTENTS

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 shall 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 2012 and for each of the years ended December 31, 2013, 2012 and 2011 have been derived from our the audited consolidated financial statements and notes thereto set forth in Item 18 of this annual report. The selected consolidated financial data as of December 31, 2011, and 2010 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.

	YEARS ENDED DECEMBER 31		
€ in thousands (except share and per share data)	**2011**	**2012**	**2013**
License revenues	—	—	440
Collaboration revenues	—	—	2,503
Total revenues	—	—	2,943
Cost of goods sold	—	—	(800)
Other income	2,192	649	585
Research and development expenses	(15,500)	(10,231)	(13,182)
Selling, general and administrative expenses	(3,807)	(4,564)	(11,628)
Other losses, net	(26)	(45)	(453)
Total Operating Costs	(19,333)	(14,840)	(25,263)
Operating result	(17,141)	(14,191)	(22,535)
Finance income	277	22	102
Finance expense	(436)	(547)	(4,387)
Finance income/(expense)—net	(159)	(525)	(4,285)
Result before corporate income taxes	(17,300)	(14,716)	(26,820)
Corporate income taxes	—	—	—
Net Loss	(17,300)	(14,716)	(26,820)
Items that may be subsequently reclassified to profit or loss	—	—	12
Other comprehensive income	—	—	—
Total comprehensive loss*	(17,300)	(14,716)	(26,808)
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)

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

The total number of ordinary shares outstanding at December 31, 2011, 2012 and 2013 was 4,749,625; 9,653,495 and 12,194,906, respectively. The share capital at December 31, 2011, 2012 and 2013 was €237,000; €483,000 and €610,000, 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
Cash and cash equivalents	17,859	1,100	263	23,810
Total assets	22,703	5,804	5,567	38,969
Total debt	4,621	4,544	1,498	7,864
Accumulated deficit	(88,205)	(105,505)	(117,234)	(144,041)
Total shareholders' equity (deficit)	13,659	(2,593)	(448)	5,564

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 €0.725 to $1.00, the official exchange rate quoted by the European Central Bank at the close of business on December 31, 2013. As of April 24, 2014, the official exchange rate of Euro to US dollars was 0.724 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.

	Period-end	Average for period	Low	High
	(€ per U.S. dollar)			
Year Ended December 31				
2010	0.748	0.754	0.687	0.837
2011	0.773	0.718	0.672	0.776
2012	0.758	0.778	0.743	0.827
2013	0.725	0.753	0.724	0.783
2014 (through April 24)	0.724	0.725	0.721	0.729
Month Ended				
October 31, 2013	0.733	0.733	0.724	0.741
November 30, 2013	0.735	0.741	0.735	0.748
December 31, 2013	0.725	0.730	0.724	0.739
January 31, 2014	0.740	0.735	0.731	0.740
February 28, 2014	0.724	0.732	0.724	0.741
March 31, 2014	0.725	0.723	0.717	0.728

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 have incurred significant losses to date. We had a net loss of €26.8 million in 2013, €14.7 million in 2012 and €17.3 million in 2011. As of December 31, 2013, we had an accumulated deficit of €144.0 million. To date, we have financed our operations primarily through the sale of equity securities and convertible debt and, to a lesser extent, through milestone payments, subsidies and grants from governmental agencies and fees for services. We have devoted substantially all of our financial resources and efforts to research and development, including preclinical studies and clinical trials. Our product, Glybera, received marketing approval under exceptional circumstances from the European Commission in October 2012. We plan in the future to apply for marketing approval for Glybera in the United States and other countries and will be required to conduct one or more additional clinical trials of Glybera. We are still in the early stages of development of the other product candidates in our pipeline. We expect to continue to incur significant expenses and losses over the next several years. Our net losses may fluctuate significantly from quarter to quarter and year to year. We anticipate that our expenses will increase substantially as we:

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

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

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

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

We anticipate that our collaborator Chiesi will commercially launch Glybera in the European Union in mid 2014 and that revenues from sales of Glybera will be one of the principal sources of funds for our business for at least the next several years, although such revenues may be modest initially. A number of factors, some of which are out of our control, may adversely affect the commercial success of Glybera, including the following:

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

Because our business is currently dependent on Glybera, failure to achieve anticipated revenues from this product would have an adverse effect on our results of operations and cause the value of our ordinary shares to decline.

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

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

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

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

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 to the extent that 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 shareholders of ordinary shares. If we raise additional funds through collaborations, strategic alliances or marketing, distribution or licensing arrangements with third parties, we may have to relinquish valuable rights to our technologies, future revenue streams, products or product candidates or grant licenses on terms that may not be favorable to us. If we are unable to raise 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.

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

As of December 31, 2013, we had a liability of €7.5 million ($10.0 million) of outstanding borrowings under our Loan and Security Agreement with Hercules, which we are required to repay in monthly installments through October 1, 2016. We could in the future incur additional debt obligations beyond our borrowings from Hercules. Our existing loan obligations, together with other similar obligations that we may incur in the future, could have significant adverse consequences, including:

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

We may not have sufficient funds, and may be unable to arrange for additional financing, to pay the amounts due under our existing loan obligations. Failure to make payments or comply with other covenants under our existing debt could result in an event of default and acceleration of amounts due. Under our agreement with Hercules, the occurrence of an event that would reasonably be expected to have a material adverse effect on our business, operations, assets or condition is an event of default. If an event of default occurs and the lender accelerates the amounts due, we may not be able to make

accelerated payments, and the lender could seek to enforce security interests in the collateral securing such indebtedness, which includes substantially all of our assets.

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

At the end of 2011, following the initial rejection of our application for marketing approval for Glybera in the European Union, our predecessor entity, Amsterdam Molecular Therapeutics, or AMT, initiated a strategic restructuring in order to conserve resources and improve its financial position. As part of this effort, AMT significantly reduced personnel, programs and spending. As a result, we lost many talented employees, including employees with an extensive understanding of our clinical programs as well as our regulatory and financial affairs. In the fourth quarter of 2011, total staff was reduced from 92 to 49. Since that time, we have hired a number of new staff, and total employee headcount as of December 31, 2013 was 87. In addition, we have engaged 33 consultants and contract workers. Nevertheless, this loss of talent and institutional knowledge has adversely affected our operations during the past year and may result in delays in preparing regulatory filings, completing clinical trials and other related activities, and could negatively impact our future business operations.

Risks Related to the Development of Our Product Candidates

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

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

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

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

We may encounter substantial delays in 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.

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 is typically required in the case of other therapies.

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 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 vector variants, such as AAV5 or AAV2. Also, while Glybera is injected directly into the muscles of the leg, the rest of the products in our pipeline target other tissues. Due to these variations, trials for our other product candidates may be less successful than the trials for Glybera.

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

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

Glybera or our product candidates may prove to have undesirable or unintended side effects, toxicities or other characteristics that may preclude our obtaining additional marketing approval or prevent or limit commercial use. In our clinical development program for Glybera, there were a total of 48 serious adverse events, two of which were determined to be related to Glybera, a pulmonary embolism and fever. In our partner's clinical development program for AIP, there was one serious adverse event that was determined by the investigator not to be treatment-related.

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 the Regulatory Approval of Glybera and Our Product Candidates

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 EU, and any approval we receive may be for a more narrow indication than we expect.

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

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

The process of obtaining marketing approval for our product candidates in the European Union, the United States and other countries is expensive and may take many years, if approval is obtained at all. 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.

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

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

product manufactured at our facility in the Netherlands and if we intend to market only product manufactured in Lexington in the United States.

Delays in designing and completing a comparability study to the satisfaction of the FDA could delay or preclude our development and commercialization plans and, thereby, limit our revenues and growth. For example, for Glybera, we may attempt to show comparability of the product manufactured at the different facilities through the use of non-clinical data, such as potency assays and animal studies. 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 requirements in the European Union, and any of our product candidates for which we obtain marketing approval in the future could be subject to similar post-approval or other regulatory requirements. Such requirements 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. 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, comply with certain notification obligations and undergo annual reassessment, the outcome of which could eventually 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.

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

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, 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 our products' status as gene therapies.

Glybera has been evaluated as a gene therapy by the EMA. We believe that all of our current product candidates, including Glybera, will be viewed as gene therapy products by the EMA, FDA and

other regulatory authorities. Gene therapies are relatively new treatments and regulators do not have extensive experience or standard review and approval processes for gene therapies. The FDA has never approved a gene therapy product as safe and effective and, unlike the EMA, does not have an exceptional circumstances approval pathway. The EMA has approved only one gene therapy, Glybera, for a subset of LPLD patients, under exceptional circumstances, and only did so by a vote of 17 to 15 and after twice denying approval.

The EMA and FDA have demonstrated caution in their regulation of gene therapy treatments, and ethical and legal concerns about gene therapy and genetic testing may result in additional regulations or restrictions on the development and commercialization of our product candidates that are difficult to predict. For example, in 2003, the FDA suspended 27 gene therapy trials involving several hundred patients after learning that a child treated in France had developed a condition resembling leukemia. Although the FDA was not aware that any of the patients treated in the 27 American trials had suffered illnesses similar to that of the infant in France, it nevertheless took precautions. This temporary halt, the largest such action involving gene therapy trials, was a setback for the field. 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 any gene therapy product not being approved.

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, that could result in changes in the data we need to submit to the EMA in order for our product candidates to gain regulatory approval. In addition, divergent scientific opinions among the various bodies involved in the review process may result in delays and require additional resources and may ultimately result in rejection. For further discussion about the regulation we face in Europe and the United States, please see "Business—Government Regulation and Reimbursement."

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. In the United States, the FDA has established the Office of Cellular, Tissue and Gene Therapies within the Agency's Center for Biologics Evaluation and Research, or CBER, to consolidate the review of gene therapy and related products, and the Cellular, Tissue and Gene Therapies Advisory Committee to advise CBER on its review. Gene therapy clinical trials conducted at institutions that receive funding for recombinant DNA research from the NIH are also subject to review by the NIH Office of Biotechnology Activities' Recombinant DNA Advisory Committee, or the RAC. 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.

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

Regulatory authorities in some jurisdictions, including the European Union and the United States, may designate drugs for relatively small patient populations as orphan drugs. Generally, if a product with an orphan drug designation subsequently receives the first marketing approval for the indication for which it has such designation, the product is entitled to a period of market exclusivity, which precludes the EMA or FDA from approving another marketing application for the same drug for the same indication for that time period. However, the same drug can subsequently be approved for the same condition if the competent regulatory agency concludes that the later drug is clinically superior in that it is shown to be safer, more effective or makes a major contribution to patient care..

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 be precluded from obtaining marketing authorization or we may lose out on the potential benefits of market exclusivity.

Risks Related to the Commercialization of Glybera and Our Product Candidates

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

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

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

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

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

Our projections of the number of people who have the diseases we are seeking to treat, as well as the subset of people with these diseases who have the potential to benefit from treatment with Glybera or our product candidates, are estimates based on our knowledge and understanding of these diseases. The total addressable market opportunity for Glybera and our product candidates will ultimately depend upon a number of factors including the diagnosis and treatment criteria included in the final label, if approved for sale in specified indications, acceptance by the medical community, patient access and product pricing and reimbursement.

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

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

Doctors may be reluctant to accept a gene therapy as a treatment option or, where available, choose to continue to rely on existing symptomatic treatments. The degree of market acceptance of Glybera, as well as of any of our product candidates that receive marketing approval in the future, will depend on a number of factors, including:

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

In the case of Glybera in the European Union, we are required to put in place a restricted access program to ensure that the product is used appropriately when the diagnosis is confirmed, mandating that the product only be supplied to doctors who have received the appropriate educational materials

and only be used to treat patients participating in a registry to monitor the outcome of patients treated with Glybera. 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 countries covered by our partnership with Chiesi.

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

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

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

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

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

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

Our commercial opportunity could be reduced or eliminated if our competitors develop and commercialize products that are safer, more effective, have fewer or less severe side effects, are more convenient or are less expensive than the products that we develop. Our competitors also may obtain EMA, FDA or other regulatory approval for their products more rapidly than we do, which could result in our competitors establishing a strong market position before we are able to enter the market. Because we expect that gene therapy patients may generally require only a single administration, we believe that the first gene therapy product to enter the market for a particular indication will likely enjoy a significant commercial advantage, and may also obtain market exclusivity under applicable orphan drug regimes.

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

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

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

We have entered into collaborations with other companies and academic research institutions with respect to important elements of our commercial and development programs. For example, we have collaboration agreements with Chiesi, for both commercialization of Glybera in the European Union and certain other countries and co-development and commercialization of our hemophilia B program, and development programs with Digna Biotech, Institut Pasteur and UCSF.

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

- collaborators have significant discretion in determining the efforts and resources that they will apply to these collaborations;
- in our current collaborations, we generally have limited or no control over the design or conduct of clinical trials sponsored by our collaborators;
- if our collaborators do not conduct the clinical trials they sponsor in accordance with regulatory requirements or stated protocols, we will not be able to rely on the data produced in such trials in our further development efforts;
- collaborators may not perform their obligations as expected;
- collaborators may also have relationships with other entities, some of which may be our competitors;
- collaborators may not pursue development and commercialization of any product candidates or may elect not to continue or renew development or commercialization programs based on clinical trial results, changes in the collaborators' strategic focus or available funding, or external factors, such as an acquisition, that divert resources or create competing priorities;

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

If our collaborations do not result in the successful development and commercialization of products or if one of our collaborators terminates its agreement with us, we may not receive 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 herein also apply to the activities of our development collaborators.

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

We believe collaborations enable us to gain access to early-stage clinical programs and related data, as well as to promising transgenes and other intellectual property, with limited financial investment by us. Part of our strategy is to leverage our experience and expertise in gene therapy research and development, as well as our proprietary manufacturing capabilities, to be an attractive collaborator for academic research institutions and biotechnology and pharmaceutical companies seeking to advance their programs into larger, late-stage clinical trials that require commercial-scale manufacturing. We face significant competition 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 in the facility we are currently building out in Lexington, Massachusetts. The insect-cell based manufacturing process we use to produce Glybera and our other product candidates is highly complex and in the normal course is subject to production difficulties. 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. Additionally, a number of factors could cause production interruptions, including equipment malfunctions, facility contamination, labor problems, raw material shortages or contamination, natural disasters, disruption in utility services, terrorist activities, human error or disruptions in the operations of our suppliers.

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

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

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

We are expending significant funds for the build-out of our leased 53,000 square foot manufacturing facility in Lexington, Massachusetts. This project may result in unanticipated delays and

cost more than expected due to a number of factors, including regulatory requirements. If construction or regulatory approval of our new facility is delayed, we may not be able to manufacture sufficient quantities of Glybera or our product candidates, which would limit our commercialization and development activities and our opportunities for growth. Cost overruns associated with this facility could also require us to raise additional funds from external sources, which may be unavailable on favorable terms or at all.

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

Our manufacturing facility in Amsterdam is, and our new facility in Lexington will be, subject to ongoing regulation and periodic inspection by the EMA, FDA and other regulatory bodies to ensure compliance with current Good Manufacturing Practices, or cGMP. Any failure to follow and document our adherence to such cGMP regulations or other regulatory requirements may lead to significant delays in the availability of products for commercial 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 will be subject to federal, state and local laws and regulations in the United States, and are subject to comparable regulations in the Netherlands, governing the use, manufacture, distribution, storage, handling, treatment and disposal of these materials. In addition to ensuring the safe handling of these materials, applicable requirements require increased safeguards and security measures for many of these agents, including controlling access and screening of entities and personnel who have access to them, and establishing a comprehensive national database of registered entities. In the event of an accident or failure to comply with environmental, occupational health and safety and export control laws and regulations, we could be held liable for damages that result, and any such liability could exceed our assets and resources.

Risks Related to Our Intellectual Property

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

We currently are heavily reliant upon licenses of proprietary technology from third parties that is important or necessary to the development of our technology and products, including technology related to our manufacturing process, our vector platform, our gene cassettes and the therapeutic genes of interest we are using. These and other licenses may not provide adequate rights to use such

technology in all relevant fields of use. Licenses to additional third-party technology that may be required for our development programs may not be available in the future or may not be available on commercially reasonable terms, which could have a material adverse effect on our business and financial condition.

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

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

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

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

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

We in-license intellectual property from third parties that is material to Glybera and all of our product candidates, including technology related to our manufacturing process, our vector platform, and the therapeutic gene cassettes we are using. Our licensing arrangements with third parties impose diligence, development and commercialization timelines, milestone payment, royalty, insurance and

other obligations on us. If we fail to comply with these obligations, our counterparties may have the right to terminate these agreements, in which case we might not be able to develop, manufacture or market any product that is covered by these agreements or may face other penalties under the agreements. Such an occurrence could materially adversely affect the value of the product candidate being developed under any such agreement. Termination of these agreements or reduction or elimination of our rights under these agreements may result in our having to negotiate new or amended agreements with less favorable terms, or cause us to lose our rights under these agreements, including our rights to important intellectual property or technology.

If we are unable to obtain and maintain patent protection for our technology and products, 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 patent 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 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. In addition, we could be found liable for monetary damages, including treble damages and attorneys' fees if we are found to have wilfully infringed a patent. A finding of infringement could prevent us from commercializing our product candidates or force us to cease some of our business operations, which could materially harm our business. Claims that we have misappropriated the confidential information or trade secrets of third parties could have a similar negative impact on our business.

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 addition, we or our collaborators may elect to reduce the price of our products in order to increase the likelihood of obtaining reimbursement approvals. In the event that countries impose prices which are not sufficient to allow us or our collaborators to generate a profit, we or our collaborators may refuse to launch the product in such countries or withdraw the product from the market. If pricing is set at unsatisfactory levels, or if the price decreases, our business could be harmed, possibly materially. If we fail to obtain and sustain an adequate level of

coverage and reimbursement for our products by third party payors, our ability to market and sell our products would be adversely affected and our business would be harmed.

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

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

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

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, the U.S. Foreign Corrupt Practices Act and certain state and local laws applicable to pharmaceutical companies.

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

Risks Related to Employee Matters and Managing Growth

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

We are highly dependent on the research and development, clinical and business development expertise of our Chief Executive Officer, Jörn Aldag, our Chief Medical Officer, Christian Meyer, M.D., and our 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 plan to expand our key capabilities and, as a result, may encounter difficulties in managing our growth, which could disrupt our operations. If we are unable to establish such capabilities we may not be successful in commercializing Glybera or our other product candidates in the United States or other countries, even if we receive marketing approval.

If we receive marketing approval, we intend to build a sales, marketing and medical affairs infrastructure to market Glybera and potentially other product candidates in the United States and other countries. We currently have no experience building and training an internal sales force. We expect in the future to experience significant growth in the number of our employees and the scope of our operations, particularly in the areas of manufacturing, clinical development, regulatory affairs and sales, marketing and distribution. To manage our anticipated future growth, we will be required to implement and improve our managerial, operational and financial systems, expand our facilities and recruit and train additional qualified personnel. Recruiting and training a sales force is expensive and time-consuming and could delay any ultimate launch of Glybera or other product candidates for which we are able to obtain marketing approval in the United States and other markets. Due to our limited financial resources and the limited experience of our management team in running a company with this level of anticipated growth, we may not be able to effectively manage the expansion of our operations or recruit and train additional qualified personnel. If 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. Since initial trading of our ordinary shares began on the NASDAQ Global Select Market on February 4, 2013 through April 23, 2014, the sale price of our ordinary shares has ranged from a high of $18.05 to a low of $8.90. 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 the ability to control all 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 50% 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.

The sale of a substantial number of our ordinary shares following expiration of lockup agreements to which certain shares are subject, may cause the market price of our ordinary shares to decline.

Sales of a substantial number of shares in the public market may occur at any time after the expiration of the lock-up agreements entered into by certain shareholders in connection with our recent initial public offering. The sale or the resale by our shareholders of such shares, or a market expectation of such sales, may cause the market price of our ordinary shares to decline. Currently, 13,434,612 ordinary shares, or 76% of our outstanding shares, are currently restricted as a result of securities laws or lock-up agreements but will be able to be sold, subject to any applicable volume limitations under U.S. federal securities laws with respect to affiliate sales and subject to the expiration of the relevant lock-up agreements, in the future.

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

As a public company, and particularly after we are no longer an emerging growth company, we will incur significant legal, accounting and other expenses that we did not incur as a private company. We currently estimate that we will incur incremental annual costs of approximately $1.5 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 that closed February 10, 2014, we were a private company with limited accounting personnel and other resources with which to address our internal controls and procedures. Our independent registered public accounting firm has not conducted an audit of our internal control over financial reporting. However, in connection with the audit of our consolidated financial statements as of and for year ended December 31, 2012 and 2013, we and our independent registered public accounting firm identified three material weaknesses in our internal control over financial reporting. A significant deficiency is a control deficiency, or a combination of control deficiencies, that adversely affects our ability to initiate, authorize, record, process, or report external financial data reliably in accordance with IFRS such that there is more than a remote likelihood that a misstatement of our annual or interim financial statements that is more than inconsequential will not be prevented or detected by our employees. A material weakness is a significant deficiency, or combination of significant deficiencies, that results in more than a remote likelihood that a material

misstatement of our annual or interim financial statement will not be prevented or detected by our employees. In response, we have begun the process of evaluating our internal control over financial reporting. We have also taken several remedial actions to address these material weaknesses. For details, see "Operating and Financial Review and Prospects—Internal Control Over Financial Reporting."

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

During the course of documenting and testing our internal control procedures in order to satisfy the requirements of Section 404, we may identify other weaknesses and deficiencies in our internal control over financial reporting. In addition, if we fail to maintain the adequacy of our internal control over financial reporting, as these standards are modified, supplemented or amended from time to time, we may not be able to conclude on an ongoing basis that we have effective internal control over financial reporting in accordance with Section 404. If we fail to achieve and maintain an effective internal control environment, we could experience material misstatements in our financial statements and fail to meet our reporting obligations, which would likely cause investors to lose confidence in our reported financial information. This could in turn limit our access to capital markets, harm our results of operations, and lead to a decline in the trading price of our ordinary shares. Additionally, ineffective internal control over financial reporting could expose us to increased risk of fraud or misuse of corporate assets and subject us to potential delisting from The NASDAQ Global Select Market, regulatory investigations and civil or criminal sanctions. We may also be required to restate our financial statements for prior periods.

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

The majority of our managing directors, supervisory directors and senior management reside outside the United States. As a result, it may not be possible for 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.

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

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 islocated at Meibergdreef 61, Amsterdam 1105 BA, the Netherlands, and our telephone number is +31 20 566 7394. Our website address is www.uniqure.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 developed Glybera, the first and currently the only gene therapy product to receive regulatory approval in the European Union. We are developing a pipeline of additional gene therapies through multiple collaborations that are designed to accelerate the development and commercialization of these programs. Our pipeline includes product candidates targeting diseases for which either the efficacy of existing treatments is limited or the administration regimen is burdensome, such as hemophilia B, as well as diseases for which there are currently no treatments, such as Sanfilippo B syndrome. We initially intend to focus on orphan diseases but believe that we will also be able to leverage our technology to develop gene therapies targeting chronic and degenerative diseases that affect larger populations. We develop our gene therapies using

our innovative, modular technology platform, which consists of a suite of components that may be applied to multiple gene therapies and includes our proprietary, cost-effective manufacturing process.

Our Gene Therapy Platform

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

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

- ***AAV-based vector delivery system.*** We deliver the gene cassette to the target tissue using an engineered, non-replicating viral vector delivery system based on AAV, a common virus that affects humans but does not cause disease. We believe that AAV is the vector of choice for most *in vivo* gene therapy applications, such as ours, in which the functional gene is introduced directly into the patient's body. We use different variants, or serotypes, of AAV, including AAV1, AAV2 and AAV5, each of which selectively targets particular tissues. In 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 recently formed, private biotechnology company with a team that we believe is a leader in AAV vector discovery and optimization. 4D uses directed evolution techniques, which involve an iterative selection process in which researchers screen libraries of mutant AAV variants to identify those that are expected to have optimal properties for achieving higher levels of gene expression. In more than 80 gene therapy clinical studies conducted by us or third parties, AAV-based vectors raised no material safety concerns. AAV-based vectors have also demonstrated sustained expression in target tissue in non-human primates for more than five years. In the hemophilia B Phase I/II clinical trial described below, St. Jude Children's Research Hospital in Memphis, Tennessee, or St. Jude, has reported expression in target tissue in humans for more than three years after a single treatment.

- ***Administration technologies.*** We and our collaborators are developing expertise in utilizing a variety of administration technologies to optimize the introduction of our gene therapy vectors to effectively deliver the transgene into the tissues and organs relevant to the indications we are targeting.

- ***Scalable, proprietary manufacturing process.*** We produce our AAV-based vectors in our own facilities with our proprietary manufacturing process, which uses insect cells and baculoviruses, a common family of viruses found in invertebrates. We believe that our manufacturing facility in Amsterdam, which the European Medicines Agency, or EMA, has approved for clinical and

commercial grade production, and our facility near Boston, Massachusetts, which we are currently building out and equipping, will enable us to produce Glybera and other gene therapies cost-effectively at commercial scale.

Glybera

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.

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

Planned U.S. Program for Glybera

We met with the FDA in August and December 2013 to discuss the regulatory pathway in the United States for Glybera. In contrast with the European Union, the United States does not have a process to approve marketing of a drug under exceptional circumstances. In our meetings, the FDA advised that it would require data in addition to what we had submitted to obtain marketing approval for Glybera in the European Union. The FDA advised that severe hypertriglyceridemia is currently considered a hallmark of LPLD, and agreed that changes in chylomicron metabolism following a meal 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 adequate for obtaining marketing approval in the United States at this stage, since it is not yet sufficiently understood how this biological

effect translates into clinical meaningfulness. The FDA recommended that we identify the clinical manifestations of LPLD for which Glybera might have the best prospects for demonstrating a meaningful impact in designing an adequate and appropriately controlled trial.

We plan to discuss the details of the EU post-approval trial and patient registry with the FDA, and if applicable to seek to amend the protocols for the post-approval trial and patient registry so that they could also serve as a clinical program with a design that addresses the FDA's requirements. We also plan to file an IND with the FDA for Glybera in the first half of 2014 so that we can include U.S. LPLD patients in the post-approval trial and registry. We believe the patient registry will provide valuable data for the FDA to consider as part of the totality of our U.S. regulatory submissions. Our current expectation, subject to satisfactory completion of regulatory discussions with the FDA, is to have sufficient data from a further clinical trial of Glybera and the patient registry to file a BLA for Glybera with the FDA in 2017.

Glybera Commercialization Plan

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

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

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

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

Product and Development Pipeline

In addition to Glybera, our development pipeline includes our internal program for hemophilia B, two collaborator-sponsored programs for monogenic diseases, one collaborator-sponsored program for a chronic degenerative disease and several programs in early preclinical development.

Our most advanced pipeline programs include the following:

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

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

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

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

- ***AMT-021 for Acute Intermittent Porphyria.*** We and our collaborator Digna Biotech are developing AMT-021 as a gene therapy for acute intermittent porphyria, or AIP, a severe liver disorder. AMT-021 consists of an AAV5 vector carrying a therapeutic porphobilinogen deaminase, or PBGD, gene that we exclusively license from Applied Medical Research Center of the University of Navarra in Spain. Our collaborator Digna Biotech is currently conducting a Phase I clinical trial of AMT-021 in eight patients in Spain. We have manufactured the gene therapy being used in this clinical trial. We understand that, to date, Digna has not observed a reduction in the urinary levels of toxic metabolites in trial participants that might have served as a surrogate marker for efficacy. We understand from Digna Biotech that clinical outcomes data are expected in the second half of 2014. Under an agreement with Digna Biotech, we have an exclusive right to use all preclinical and Phase I clinical trial data from this program.

- ***AMT-110 for Sanfilippo B Syndrome.*** We and our collaborator Institut Pasteur are developing AMT-110 as a gene therapy for Sanfilippo B syndrome, a potentially fatal lysosomal storage

disease that results in serious brain degeneration in children. This gene therapy consists of an AAV5 vector carrying a therapeutic a-N-acetylglucosaminidase, or NaGLU, gene. Our collaborator Institut Pasteur is currently conducting a Phase I/II clinical trial of AMT-110 in four patients in France. We have manufactured the gene therapy being used in this clinical trial. We have an agreement in principle with Institut Pasteur to acquire the clinical results and commercial rights under this program following completion of this Phase I/II clinical trial, and are currently in negotiations with Institut Pasteur regarding the terms of a definitive agreement in this regard. We understand from Institut Pasteur that data are expected in the first half of 2015. We believe that if the results of this clinical trial are positive, it will constitute proof of concept of the administration to the brain of a gene therapy for lysosomal storage diseases.

- ***AAV2/GDNF for Parkinson's Disease.*** We and our collaborator the University of California at San Francisco, or UCSF, are developing 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 and will involve 24 patients. UCSF's product candidate has been manufactured by a third party using a mammalian cell-based process. In this clinical trial, the NIH is administering the gene therapy using convection enhanced delivery, which is a process developed by UCSF with the goal of achieving more precisely targeted administration than the methods used in earlier approaches, which may result in improved efficacy. We have a license under UCSF's rights to use all preclinical and clinical data from the UCSF program for any future development program. Based on the results of the UCSF program, we may decide to develop an AAV2-based gene therapy containing the GDNF gene manufactured with our insect cell-based manufacturing process.

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

Our Strategy

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

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

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.

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:

- 13 patent families that we own;
- 8 patent families that we exclusively in-license; and
- 6 patent families that we non-exclusively in-license.

The geographic breakdown of our owned patent portfolio is as follows:

- 2 issued U.S. patents;
- 2 granted European Patent Office patents;
- 1 pending PCT patent application;
- 7 pending U.S. patent applications;
- 8 pending European Patent Office patent applications; and
- 57 pending patent applications in other jurisdictions.

The patent portfolios for our manufacturing platform and most advanced programs are summarized below.

NIH Patents

Our manufacturing patent families contain issued patents in the United States, Europe and other territories, as well as numerous pending patent applications.

We have non-exclusively in-licensed from the NIH a patent family relating to the insect cell-based manufacturing of AAV-based vectors. The patents in this family include two issued patents in the United States and one issued patent in Europe, as well as issued patents in other jurisdictions. The standard 20-year term for patents in this family will expire in 2022. This patent family relates to technology used in Glybera and all of our development programs.

We also in-license from the NIH two patent families relating to AAV5-based vectors. These patents are licensed exclusively for AAV5-based therapeutic products to be delivered to the brain or liver for the treatment of human diseases originating in the brain or liver, excluding arthritis-related diseases, and non-exclusively for AAV5-based therapeutic products to treat any human disease in any manner not covered by the exclusive license. The patents in the first family include two issued patents in the United States, one issued patent in Europe and two issued patents in Japan, as well as issued patents and a pending application in other jurisdictions. The standard 20-year term for patents in this family will expire in 2019. This patent family relates to technology used in our AIP, hemophilia B and Sanfilippo B programs. The second family includes one issued U.S. patent with a standard 20-year term that will expire in 2020. This patent family relates to technology used in our Sanfilippo B program. See "Risk Factors—Risks Related to Our Intellectual Property—Our intellectual property licenses with third parties may be subject to disagreements over contract interpretation, which could narrow the scope of our rights to the relevant intellectual property or technology or increase our financial or other obligations to our licensors."

Other Manufacturing Patents

We own a patent family directed to improved AAV vectors that are stably expressed in insect cells. The family includes an issued patent in the United States and pending applications in the United States, Europe, Japan and other jurisdictions. The standard 20-year term for patents in this family will expire in 2027. This patent family relates to technology used in Glybera and all of our development programs.

We also in-license a patent family related to aspects of the AAV insect cell production technology from Protein Sciences Corporation. This family includes issued patents in the United States, Europe and elsewhere. This license is exclusive in respect of the products we develop with the use of this patent family for LPLD, hemophilia B and AIP, and we may add additional products to the license on an exclusive basis except in certain specified circumstances. The standard 20-year term for patents in

this family will expire in 2019. This patent family relates to technology used in Glybera and all of our development programs.

We non-exclusively in-license a family of patents relating to methods for intramuscular administration of AAV vectors from Asklêpios Biopharmaceutical, Inc., or AskBio. This family includes issued patents in Europe, Japan and other jurisdictions, and a pending application in the United States. The standard 20-year term for patents in this family will expire in 2016. This patent family relates to technology used in Glybera.

We own a method of manufacturing patent family relating to a second- generation manufacturing method used in our AIP, hemophilia B and Parkinson's disease programs. This patent family contains pending applications in the United States, Europe, Japan and other jurisdictions, and issued patents in several jurisdictions. The standard 20-year term for patents in this family will expire in 2028.

We also own a PCT application that relates to a proprietary baculovirus filtration process. The standard 20-year term for patents in this family, if issued, will expire in 2032. This patent family relates to technology used in Glybera and all of our development programs.

Glybera

We co-own with University of British Columbia, or UBC, a patent family relating to the lipoprotein lipase variant LPL-S447X transgene used in Glybera, including issued patents in Europe and Japan. The standard 20-year term for patents in this family will expire in 2020. UBC exclusively licensed its patent rights to Xenon, which has granted us the sublicense described below.

We exclusively in-license from Aventis Pharma S.A., or Aventis, a patent family co-owned by UBC and Aventis that relates to the use of AAV-LPL vectors for LPL-deficiency, including issued patents in Europe and other jurisdictions and two pending U.S. patent applications. The standard 20-year term for patents in this family will expire in 2015.

We own a family of patents relating to a VP1 vector capsid modification, which relates to the production of AAV vectors in insect cells and to AAV vectors with an altered ratio of viral capsid proteins that provides improved infectivity of the viral particles. This patent family includes issued patents in the United States, Europe and elsewhere, as well as pending applications in Europe, Japan and other jurisdictions. The standard 20-year term for patents in this family will expire in 2026.

We non-exclusively in-license a patent family from the Salk Institute that relates to a genetic promoter that enhances the expression of LPL- S447X delivered to the target tissues. This family includes four issued patents in the United States that have standard 20-year terms that will expire in 2017, and issued patents in Europe and other jurisdictions that have standard 20-year terms that will expire in 2018.

We non-exclusively in-license a patent family relating to the AAV1 capsid from AmpliPhi Biosciences, Inc. (formerly Targeted Genetics Corporation), or AmpliPhi. This family includes three issued patents in the United States, and one each in Europe and Japan, as well as issued patents elsewhere and a pending application in the United States. The standard 20-year term for patents in this family will expire in 2019. The University of Pennsylvania exclusively licensed its patent rights to AmpliPhi, which has granted us the sublicense described below.

Other Programs

Hemophilia B. Our patent portfolio covering our hemophilia B program includes an exclusively in-licensed patent family from St. Jude relating to a specific promoter and a codon optimized hFIX transgene. This patent family includes two issued patents in the United States and one in Europe. The 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 pending applications in the United States, Europe, Japan and elsewhere. The standard 20-year term for patents in this family will expire in 2029.

Parkinson's disease. For our Parkinson's disease program, we have in- licensed a patent family and corresponding know-how relating to the GDNF transgene from Amgen for the field of gene therapy. The license is exclusive and expires on a country-by-country basis on the later of 10 years following launch of the relevant product or of expiration of the last- to-expire licensed patent in the applicable country, after which the license will become non-exclusive for that given country. This patent family includes two issued patents in the United States, one of which will expire in 2015 and one in 2017.

Licenses

We have obtained exclusive or non-exclusive rights from third parties under a range of patents and other technology that we use in our product and development programs, as described below. Our agreements with these third parties generally grant us a license to make, use, sell, offer to sell and import products covered by the licensed patent rights in exchange for our payment of some combination of an upfront amount, annual fees, royalties, a portion of amounts we receive from our licensees and payments upon the achievement of specified development, regulatory or commercial milestones. Some of the agreements specify the extent of the efforts we must use to develop and commercialize licensed products. The agreements generally expire upon expiration of the last-to-expire valid claim of the licensed patents. Each licensor may terminate the applicable agreement if we materially breach our obligations and fail to cure the breach within a specified cure period, in addition to other termination rights in some cases.

Technology Used for Multiple Programs

We are exploiting technology from the third party sources described below in more than one of our programs.

National Institutes of Health—AAV production. In 2007, we entered into a license agreement with the NIH, which we amended in 2009 and 2013. Under the license agreement, the NIH has granted us a non-exclusive license to patents relating to production of AAV vectors, to make, use, sell, offer to sell and import specified plasmids, which are small DNA molecules that are physically separate from, and can replicate independently of, chromosomal DNA within a cell, or other materials, which we refer to as AAV products. We may only grant sublicenses under this agreement with the NIH's consent, which may not be unreasonably withheld. We are exploiting this technology for our Glybera program and our programs for hemophilia B, AIP, and Sanfilippo B syndrome, and Parkinson's disease.

Payment obligations to the NIH under this license agreement include a one-time upfront payment of $12,000, which we have paid; a low single-digit percentage royalty on the sale of AAV products by us or on our behalf; a maximum sub-teen double-digit percentage of sublicensing income; potential additional development milestone fees for the initiation of each clinical trial, which would total in the aggregate $255,000 for one Phase I, Phase II and Phase III trial; potential regulatory milestone fees totaling $750,000 for the first marketing approvals in specified countries or jurisdictions; and an annual maintenance fee of $15,000 creditable against royalties. We do not have to pay royalties or milestone fees under this agreement if we have to pay royalties or milestone fees under our 2011 agreement with the NIH, described below, for the same product. In connection with entering into our relationship with Chiesi and obtaining the NIH's consent to sublicense our rights under this agreement to Chiesi, we also paid the NIH a total of $328,684 in amendment and sublicense payments. Under the license agreement,

we have agreed to meet benchmarks in our development efforts, including as to development events, clinical trials and marketing approval, within specified timeframes.

The NIH may terminate this agreement in specified circumstances relating to our insolvency or bankruptcy. We may terminate this agreement for any reason, in any territory, subject to a specified notice period.

National Institutes of Health—AAV5. In 2011, we entered into another license agreement with the NIH, which superseded a prior 2007 agreement and which we amended in 2013. Under this agreement, the NIH granted us an exclusive, worldwide license to patents relating to AAV5 for use in therapeutic products to be delivered to the brain or liver for treatment of human diseases originating in the brain or liver, but excluding arthritis-related diseases, and a non-exclusive, worldwide license to patents relating to AAV5 for all other diseases, in each case to make, use, sell, offer to sell and import products within the scope of the specified patent claims. We refer to the products licensed under this agreement as AAV5 products. We may grant sublicenses under this agreement only with the NIH's consent, which may not be unreasonably withheld. We are currently exploiting this technology for our programs on hemophilia B, AIP, and Sanfilippo B syndrome. See "Risk Factors—Our intellectual property licenses with third parties may be subject to disagreements over contract interpretation, which could narrow the scope of our rights to the relevant intellectual property or technology or increase our financial or other obligations to our licensors."

We have agreed to pay the NIH an initial payment of $140,000, which we have paid, an amendment royalty fee of $500,000, of which $250,000 would be payable upon a sublicense of the corresponding rights, which we have paid in full, royalties equal to a low single-digit percentage of net sales of AAV5 products, if any, by or on behalf of us or our sublicensees; a single to 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 partner, a low single-digit as a percentage of net sales royalty, or, if higher, a high single-digit to sub- teen double-digit royalty as a percentage of royalties we receive from such commercialization partner plus an equivalent percentage of the price we invoice the commercialization partner for the licensed products less our cost of goods sold, subject to a floor of a low single-digit percentage of net sales by Chiesi or another commercialization partner. We have also agreed to pay Aventis a one-time milestone fee of €50,000 upon our achievement of a specified regulatory milestone and €75,000 upon our achievement of a specified commercial milestone.

In conjunction with amending the agreement in 2013, we have agreed to provide Aventis with a right of first negotiation regarding a specified product candidate to treat AIP if, at the time we complete Phase I/II clinical trials of the product candidate or within a specified period thereafter, we contemplate entering into a partnership for the co-development and commercialization of the product candidate.

The agreement will remain in effect until the expiration of the protection provided by the licensed patents, or until we or Aventis exercise our rights to terminate it. Aventis may terminate the agreement in circumstances relating to our bankruptcy.

Asklêpios Biopharmaceutical. In 2010, we entered into a license agreement with AskBio under which AskBio granted us a non-exclusive, worldwide license, with a right to sublicense, to patents relating to administration of an AAV vector to muscle tissue for use in treatment of LPLD with Glybera or other products that contain an AAV vector having an AAV genetic construct encoding an LPL gene variant, to research, develop, make, use, sell, offer for sale, and import the products to treat LPLD.

We made a one-time upfront payment to AskBio of $50,000 and have agreed to pay AskBio annual maintenance fees of $50,000 during the term of the license.

The agreement will remain in effect on a country-by-country basis until the earlier of June 5, 2016 or the expiration of the last to expire of the valid claims in the licensed patents. We may terminate the agreement for convenience at any time subject to a specified notice period.

Salk Institute for Biological Studies. In 2008, we entered into a license agreement with the Salk Institute for Biological Studies, or Salk, which we amended in 2013. Under the license agreement, Salk has granted us a non- exclusive license to specified biological materials and patents relating to a DNA promoter, to research, develop, make, use, import, offer for sale, and sell products using their technology for gene therapy. We have a right to enter into sublicenses under this agreement, subject to prior written consent by Salk, which may not be unreasonably withheld, and to other conditions.

Payment obligations under the agreements include an upfront payment of $35,000 in 2008 and $5,000 in 2013 in connection with an amendment and consent to sublicense to Chiesi, both of which we have paid, as well as annual maintenance fees of $30,000, a royalty equal to a low single-digit percentage of net sales, if any, of licensed products sold by us, or, if higher, by Chiesi, and payments of

a low single-digit percentage of all execution fees, maintenance fees, milestone fees and other non-royalty payments received by us from Chiesi or any other sublicensee.

The agreement will remain in effect on a country-by-country basis until the latest of 15 years from the effective date, the date of expiration of the last to expire licensed patent or the abandonment of the last remaining licensed patent application.

AmpliPhi Biosciences. In 2006, we entered into a license agreement with AmpliPhi (formerly Targeted Genetics Corporation), which we amended in 2013. Under the license agreement, AmpliPhi has granted us a non-exclusive, worldwide sublicense to patents exclusively licensed by AmpliPhi from the University of Pennsylvania, or Penn, relating to AAV1, to make, develop, use, sell, offer to sell and import products using the patent rights to treat LPLD type 1, which includes the Glybera patient population, and LPLD type 5 by in vivo gene therapy. We may only grant sublicenses under this agreement with the consent of AmpliPhi and Penn, which may not be unreasonably withheld.

We have to date paid to AmpliPhi a one-time up-front payment of $1,750,000. We have agreed to pay AmpliPhi annual fees of $100,000, a total of $4,950,000 in development and regulatory milestone payments, and a royalty equal to a low single-digit percentage of net sales, if any, of licensed products sold by us or Chiesi.

Either party may terminate the agreement in circumstances relating to the other party's insolvency or bankruptcy. We may terminate the agreement for convenience at any time subject to a specified notice period.

If the agreement is terminated by us due to AmpliPhi's insolvency, bankruptcy or material uncured breach, or if AmpliPhi's license agreement with Penn is terminated, our license from AmpliPhi may be assigned to Penn. The assignment must be made on our request but is at Penn's discretion, which Penn may not unreasonably withhold, provided that the agreement specifies that Penn's obligations are consistent with its current obligations and provided that we assume all AmpliPhi's obligations.

Hemophilia B

St. Jude Children's Research Hospital. In 2008, we entered into a license agreement with St. Jude, which we amended in 2012. Under the license agreement, St. Jude has granted us an exclusive license, with a right to sublicense, to patent rights relating to expression of hFIX in gene therapy vectors, to make, import, distribute, use and commercialize products containing hFIX covered by a valid patent claim in the field of gene therapy for treatment or prophylaxis of hemophilia B. In addition, we have a first right of negotiation regarding any patent applications that are filed by St. Jude for any improvements to the patent rights licensed to us.

We have agreed to pay St. Jude a royalty equal to a low single-digit percentage of net sales, if any, by us or our sublicensees of products covered by the licensed patent rights, and a portion of certain amounts we receive from sublicensees ranging from a mid-single digit to a mid-teen double-digit percentage of such amounts. We have also agreed to pay St. Jude one-time milestone fees totaling $6,500,000 upon the achievement of specified development and regulatory milestones, and an annual maintenance fee of $10,000 creditable against royalties and milestones in the same year. We have agreed to use commercially reasonable efforts to diligently develop and commercialize products licensed under this agreement.

The agreement will remain in effect until no further payment is due relating to any licensed product under this agreement or either we or St. Jude exercise our rights to terminate it. St. Jude may terminate the agreement in specified circumstances relating to our insolvency. We may terminate the agreement for convenience at any time subject to a specified notice period.

AIP

Digna Biotech. In 2010, we entered into a license agreement with Digna Biotech, S.L, or Digna Biotech, Fundación para la Investigación Médica Applicada, or FIMA, the members of a collaborative research consortium known as UTE CIMA, and Proyecto de Biomedicina CIMA S.L., or Proyecto, which superseded several prior agreements amongst such parties. We refer to Digna Biotech, FIMA, UTE CIMA and Proyecto collectively as the CIMA Parties. Under the license agreement, Proyecto granted us an exclusive, worldwide license, with a right to sublicense, under its interest in patent rights we jointly own with Proyecto relating to PBGD gene therapy to use, develop, make, have made and commercialize products using the licensed patent rights. In addition, UTE CIMA granted us a non-exclusive, worldwide license, with the right to grant sublicenses, under certain patent rights, know-how and materials required for the use, development, manufacture or commercialization of products covered by our exclusive license from Proyecto in the gene therapy field.

We have agreed to pay Digna Biotech royalties equal to a mid-single digit percentage of net sales, if any, by us or our affiliates of licensed products covered by our exclusive license and a sub-teen double-digit percentage share of net revenues we receive from our sublicensees. Digna Biotech is responsible for apportioning the amounts we pay Digna Biotech amongst the CIMA Parties.

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

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

Parkinson's disease

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

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

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

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

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

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

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

The UCSF agreement also contains certain other obligations we have agreed to complete by specified dates, including obligations to deliver to UCSF by June 12, 2014 specified materials for UCSF to complete a non-clinical study of an AAV2 vector carrying the GDNF gene, to demonstrate equivalent product release specifications of our vector to the vector used in the ongoing NIH-sponsored Phase I clinical trial, to pursue a bridging study using our AAV2 vector carrying a GDNF gene, and to use commercially reasonable efforts to proceed, either directly or through a third party licensee, to develop, seek to obtain regulatory approval for and market at least one identified product in the United States and the European Union.

If we materially fail to comply with any of the diligence obligations described above and do not cure such failure within specified cure periods, UCSF may at its option either terminate the data license agreement or be freed from its covenant not to grant to any other for-profit entity any of the rights granted to us thereunder.

The data license agreement will remain in effect until all of our payment obligations to UCSF have ended in all countries, unless either we or UCSF exercise our rights to terminate it earlier. UCSF may terminate the agreement in specified circumstances relating to our bankruptcy. We may terminate the agreement for convenience at any time subject to a specified notice period.

Trade Secrets

In addition to patents and licenses, we rely on trade secrets and know-how to develop and maintain our competitive position. For example, significant aspects of the process by which we manufacture Glybera and our gene therapies are based on unpatented trade secrets and know-how. We seek to protect our proprietary technology and processes and obtain and maintain ownership of certain technologies, in part, through confidentiality agreements and invention assignment agreements with our employees, consultants, scientific advisors, contractors and commercial partners. We also seek to preserve the integrity and confidentiality of our data, trade secrets and know-how by maintaining physical security of our premises and physical and electronic security of our information technology systems.

Trademarks

uniQure and Glybera are registered trademarks in various jurisdictions including the United States and the European Union. We intend to seek trade mark protection for other product candidates as and when appropriate.

Strategic Collaboration: Chiesi

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

Under our Glybera commercialization agreement, we will receive payments from Chiesi for the quantities of Glybera we manufacture and supply to them. We are required to pay the cost of goods sold, including royalty and other payments to third parties in connection with the sale of Glybera. Based on our estimates, we anticipate we will retain in the range of 20% to 30% of the net sales of Glybera by Chiesi in the European Union and other countries under our agreement, net of the cost of goods sold, including the royalties and other obligations we owe to third parties. In addition, we are required to repay 20% of the gross amount received from Chiesi related to Glybera sales in repayment of a technical development loan from the Dutch government, which has a current outstanding balance of €5.5 million.

Under our hemophilia B co-development agreement, we will also receive payments from Chiesi for any commercial quantities of our hemophilia B product candidate we manufacture and supply to them, if we receive regulatory approval for such product candidate. We estimate that the amount we would

retain, net of cost of goods sold, including third party royalties and related amounts, will be between 25% and 35% of the revenues from sales of such product by Chiesi, varying by country of sale.

Strategic Collaboration: 4D Molecular Therapeutics

In January 2014, we entered into a collaboration and license agreement with 4D for the discovery and optimization of next-generation AAV vectors. Under this agreement, 4D has granted us an exclusive, worldwide license, with the right to grant sublicenses, to 4D's existing and certain future know-how and other intellectual property, including certain patent rights 4D has exclusively licensed from the Regents of the University of California, 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 agreed to establish a laboratory to identify next generation AAV vectors. In addition, in connection with our entry into this collaboration, Dr. Schaffer will join our Supervisory Board.

We have agreed to fund a three-year research collaboration, which can be extended at our option for an additional year, to be conducted under a mutually agreed research plan. We are entitled to select a specified number of AAV variants from the research collaboration. We will have exclusive rights to further research, develop, manufacture and commercialize the selected AAV variants, as well as AAV vectors and products containing such AAV 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 agreed to make a one-time upfront payment of $100,000 and another one-time payment of $100,000 upon the joint research steering committee's approval of the research plan, including an associated budget. Our payment obligations under the agreement include the research collaboration funding described above as well as payments for the achievement of specified pre-clinical, clinical and regulatory milestones of up to $5,000,000 for each licensed product that we develop under the collaboration, 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 will 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.

Competition

The biotechnology and pharmaceutical industries, including in the gene therapy field, are characterized by rapidly advancing technologies, intense competition and a strong emphasis on intellectual property. We face substantial competition from many different sources, including large and specialty pharmaceutical and biotechnology companies, academic research institutions and governmental agencies and public and private research institutions.

We are aware of several companies focused on developing gene therapies in various indications, including bluebird bio, Sangamo BioScience, AGTC, Oxford Biosciences, Spark Therapeutics, Audentes Therapeutics, RegenX and Baxter, as well as several companies addressing other methods for modifying genes and regulating gene expression. Although companies and research institutions in the gene

therapy field tend to focus on particular target indications, any advances in gene therapy technology made by a competitor may be used to develop therapies competing against Glybera or one of our product candidates. We may also face competition with respect to the treatment of some of the diseases that we are seeking to target with our gene therapies from protein pharmaceuticals under development at pharmaceutical and biotechnology companies such as Pfizer, Baxter, Bayer, Novo Nordisk, Genzyme, Shire, BioMarin, Biogen Idec and numerous other pharmaceutical and biotechnology firms.

We must also compete with existing standards of care, therapies and symptomatic treatments, as well as any new therapies that may become available in the future for the indications we are targeting.

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

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, pre-clinical and clinical testing, manufacture (including any manufacturing changes), packaging, storage, recordkeeping, labeling, advertising, promotion, distribution, marketing, post-approval monitoring and reporting, and import and export of pharmaceutical products, biological products and medical devices. We believe that all of our product candidates will be regulated as biological products, or biologics, and in particular, as gene therapies, and will be subject to such requirements and regulations under U.S. and foreign laws.

Regulation in the United States

In the United States, the Food and Drug Administration, or FDA, regulates biologics under the Public Health Service Act, or PHSA, and the Federal Food, Drug, and Cosmetic Act, or FDCA, and regulations and 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. The failure to comply with applicable requirements may subject an applicant and/or sponsor to a variety of administrative or judicial sanctions, including refusal by the FDA to approve pending applications, withdrawal of a license, imposition of a clinical hold, issuance of warning letters and other types of letters, product recalls, fines, and civil or criminal investigations and penalties brought by the Department of Justice and other federal and state government agencies.

All of our current product candidates are subject to regulation by the FDA as biologics. An applicant seeking approval to market and distribute a new biologic in the United States must typically undertake the following:

- completion of preclinical laboratory tests, animal studies and formulation studies in compliance with the FDA's current Good Laboratory Practice, or cGLP, regulations;
- submission to the FDA of an Investigational New Drug, or IND Application, which allows human clinical trials to begin unless the FDA objects within 30 days;
- approval by an independent institutional review board, or IRB, representing each clinical site before each clinical trial may be initiated;
- performance of adequate and well-controlled human clinical trials in accordance with the FDA's or EMA's good clinical practices, or GCP, to establish the safety, potency, purity and efficacy of the proposed biological product for each indication;
- preparation and submission to the FDA of a Biologics License Application, or BLA;
- satisfactory review of the BLA by an FDA advisory committee, when appropriate or if applicable;
- satisfactory completion of one or more FDA inspections of the manufacturing facility or facilities at which the product, or components thereof, are produced to assess compliance with cGMP requirements and to assure that the facilities, methods and controls are adequate to preserve the product's identity, strength, quality and purity;
- payment of user fees and securing FDA approval of the BLA; and
- compliance with any post-approval requirements, including Risk Evaluation and Mitigation Strategies, or REMS, and post-approval studies required by FDA.

Human Clinical Studies Under an IND

Clinical trials involve the administration of the investigational biologic to human subjects under the supervision of qualified investigators in accordance with cGCP requirements. A protocol for each clinical trial and any subsequent protocol amendments must be submitted to the FDA as part of an IND. A clinical trial may not proceed unless and until an IND becomes effective, which is 30 days after its receipt by the FDA unless before that time the FDA raises concerns or questions related to one or more proposed clinical trials and places the trial on clinical hold.

In addition, an IRB at each institution participating in the clinical trial must review and approve the plan for any clinical trial before it commences at that institution, and the IRB must conduct continuing review and reapprove the study at least annually. An IRB must operate in compliance with FDA regulations, and information about certain clinical trials must be submitted within specific timeframes to the NIH for public dissemination on their ClinicalTrials.gov website.

Human clinical trials are typically conducted in three sequential phases, which may overlap or be combined:

- Phase I: The biological product is initially introduced into healthy human subjects or patients with the target disease or condition and tested for safety, dosage tolerance, absorption, metabolism, distribution, excretion and, if possible, to gain an early understanding of its effectiveness.
- Phase II: The biological product is administered to a limited patient population to identify possible adverse effects and safety risks, to preliminarily evaluate the efficacy of the product for specific targeted diseases and to determine dosage tolerance and optimal dosage.

- Phase III: The biological product is administered to an expanded patient population in adequate and well-controlled clinical trials to generate sufficient data to statistically confirm the potency and safety of the product for approval, to establish the overall risk-benefit profile of the product and to provide adequate information for the labelling of the product.

Progress reports detailing the results of the clinical trials must be submitted at least annually to the FDA and more frequently if serious adverse events occur. The FDA or the sponsor may suspend or terminate a clinical trial at any time on various grounds, including a finding that the research subjects are being exposed to an unacceptable health risk. Similarly, an IRB can suspend or terminate approval of a clinical trial at its institution if the clinical trial is not being conducted in accordance with the IRB's requirements or if the biologic has been associated with unexpected serious harm to patients.

FDA Guidance Governing Gene Therapy Products

The FDA has issued various guidance documents regarding gene therapies, which outline additional factors that the FDA will consider at each of the above stages of development and relate to, among other things, the proper preclinical assessment of gene therapies; the chemistry, manufacturing, and control information that should be included in an IND application; the proper design of tests to measure product potency in support of an IND or BLA application; and measures to observe delayed adverse effects in subjects who have been exposed to investigational gene therapies when the risk of such effects is high.

If a gene therapy trial is conducted at, or sponsored by, institutions receiving the NIH funding for recombinant DNA research, a protocol and related documentation must be submitted to, and the study registered with, the NIH Office of Biotechnology Activities, or OBA, pursuant to the NIH Guidelines for Research Involving Recombinant DNA Molecules prior to the submission of an IND to the FDA. In addition, many companies and other institutions not otherwise subject to the NIH Guidelines voluntarily follow them. The NIH will convene the Recombinant DNA Advisory Committee (RAC), a federal advisory committee, to discuss protocols that raise novel or particularly important scientific, safety or ethical considerations at one of its quarterly public meetings. The OBA will notify the FDA of the RAC's decision regarding the necessity for full public review of a gene therapy protocol. RAC proceedings and reports are posted to the OBA web site and may be accessed by the public.

Compliance with cGMP Requirements

Manufacturers of biologics must comply with applicable cGMP regulations, including quality control and quality assurance and maintenance of records and documentation. Manufacturers and others involved in the manufacture and distribution of such products must also register their establishments with the FDA and certain state agencies. Both domestic and foreign manufacturing establishments must register and provide additional information to the FDA upon their initial participation in the manufacturing process. Establishments may be subject to periodic unannounced inspections by government authorities to ensure compliance with cGMPs and other laws. Discovery of problems may result in a government entity placing restrictions on a product, manufacturer, or holder of an approved BLA, and may extend to requiring withdrawal of the product from the market. The FDA will not approve an application unless it determines that the manufacturing processes and facilities are in compliance with cGMP requirements and adequate to assure consistent production of the product within required specification.

Submission of a BLA

The results of the preclinical and clinical studies, together with detailed information relating to the product's chemistry, manufacture, controls and proposed labeling, among other things, are submitted to the FDA as part of a BLA requesting a license to market the product for one or more indications.

Under federal law, the submission of most BLAs is subject to an application user fee, currently exceeding $2.1 million, and the sponsor of an approved BLA is also subject to annual product and establishment user fees, currently exceeding $104,000 per product and $554,600 per establishment. These fees are typically increased annually. The FDA has agreed to specified performance goals in the review of BLAs. Most such applications are meant to be reviewed within ten months from the date of filing, and most applications for "priority review" products are meant to be reviewed within six months of filing.

The FDA may also refer applications to an advisory committee for review and a vote on approval. Typically, an advisory committee includes clinicians and other experts who review, evaluate and vote on a recommendation as to whether the application should be approved. The FDA is not bound by the recommendations of an advisory committee, but it considers such recommendations carefully when making decisions.

Expedited Review

The FDA is authorized to expedite the review of BLAs in several ways. Under the fast track program, the sponsor of a biologic candidate may request the FDA to designate the product for a specific indication as a fast track product concurrent with or after the filing of the IND for the product candidate. In addition to other benefits, such as the ability to use surrogate endpoints and have greater interactions with the FDA, the FDA may initiate review of sections of a fast track product's BLA before the application is complete. FDA may also take certain actions with respect to products designated as breakthrough therapies, including holding meetings with the sponsor and the review team throughout the development process; providing timely advice to and communication with the product sponsor regarding development and approval; involving more senior staff in the review process; assigning a cross-disciplinary project lead for the review team; and taking certain steps to design the clinical trials in an efficient manner.

FDA's Decision on a BLA and Post-Approval Requirements

On the basis of the BLA and accompanying information, including the results of the inspection of the manufacturing facilities, the FDA may issue an approval letter or a complete response letter. An approval letter authorizes commercial marketing of the biological product with specific prescribing information for specific indications. A complete response letter generally outlines the deficiencies in the submission and may require substantial additional testing or information in order for the FDA to reconsider the application. If and when those deficiencies have been addressed to the FDA's satisfaction in a resubmission of the BLA, the FDA will issue an approval letter.

If the FDA approves a product, it may limit the approved indications for use of the product, require that contraindications, warnings or precautions be included in the product labeling, require that post-approval studies, including Phase IV clinical trials, be conducted to further assess a biologic's safety after approval, require testing and surveillance programs to monitor the product after commercialization, or impose other conditions, including distribution restrictions or other risk management mechanisms, including Risk Evaluation and Mitigation Strategies (REMS). The FDA may prevent or limit further marketing of a product based on the results of post-market studies or surveillance programs.

Following approval, some types of changes to the approved product, such as adding new indications, manufacturing changes and additional labeling claims, are subject to further testing requirements and the FDA review and approval. The product may also be subject to official lot release, meaning that the manufacturer is required to perform certain tests on each lot of the product before it is released for distribution. Other post-approval requirements include reporting of cGMP deviations that could affect the identity, potency, purity and overall safety of a distributed product, reporting of

adverse effects, reporting new information regarding safety and efficacy, maintaining adequate record-keeping, and complying with electronic record and signature requirements.

Biosimilars and Exclusivity

The 2010 Patient Protection and Affordable Care Act authorized the FDA to approve biosimilars. Under the Act, a manufacturer may submit an application for licensure of a biologic product that is "biosimilar to" or "interchangeable with" a previously approved biological product or "reference product." In order for the FDA to approve a biosimilar product, it must find that there are no clinically meaningful differences between the reference product and proposed biosimilar product. A finding of "interchangeability" requires that a product is determined to be biosimilar to the reference product, and that the product can be expected to produce the same clinical results as the reference product. An application for a biosimilar product may not be submitted to the FDA until four years following approval of the reference product, and it may not be approved until 12 years thereafter. These exclusivity provisions only apply to biosimilar companies and not companies that rely on their own data and file a full BLA.

Orphan Drug Exclusivity

Under the Orphan Drug Act, the FDA may designate a biological product as an "orphan drug" if it is intended to treat a rare disease or condition (generally meaning that it affects fewer than 200,000 individuals in the United States, or more in cases in which there is no reasonable expectation that the cost of developing and making a biological product available in the United States for treatment of the disease or condition will be recovered from sales of the product). If a product with orphan status receives the first FDA approval, it will be granted 7 years of market exclusivity (meaning that the FDA may not approve any other applications for the same product for the same indication for seven years, except in certain limited circumstances). Competitors may receive approval of different products for the indication for which the orphan product has exclusivity and may obtain approval for the same product but for a different indication. Orphan product designation does not convey any advantage in or shorten the duration of the regulatory review and approval process. In the European Union, we have been granted orphan drug exclusivity for Glybera for treatment of LPLD until October 2022, subject to the conditions applicable to orphan drug exclusivity. The FDA has also granted orphan drug designation to Glybera for treatment of LPLD, meaning that it will receive orphan drug exclusivity if it is the first product approved for that indication.

Pediatric Exclusivity

Pediatric exclusivity is another type of regulatory exclusivity in the United States and, if granted, provides for the attachment of an additional six months of marketing protection to the term of any existing regulatory exclusivity, including orphan exclusivity and exclusivity against biosimilars. This six-month exclusivity may be granted if a BLA sponsor submits pediatric data that fairly respond to a written request from the FDA for such data. The data do not need to show the product to be effective in the pediatric population studied; rather, if the clinical trial is deemed to fairly respond to the FDA's request, the additional protection is granted. If reports of requested pediatric studies are submitted to and accepted by the FDA within the statutory time limits, whatever statutory or regulatory periods of exclusivity cover the product are extended by six months.

FDA Regulation of Companion Diagnostics

We may seek to develop *in vitro* and *in vivo* companion diagnostics for use in selecting the patients that we believe will respond to our gene therapies. FDA officials have issued draft guidance that, when finalized, would address issues critical to developing *in vitro* companion diagnostics, such as biomarker qualification, establishing clinical validity, the use of retrospective data, the appropriate patient

population and when the FDA will require that the device and the drug be approved simultaneously. The draft guidance issued in July 2011 states that if safe and effective use of a therapeutic product depends on an *in vitro* diagnostic, then the FDA generally will require approval or clearance of the diagnostic at the same time that the FDA approves the therapeutic product. The FDA has yet to issue further guidance, and it is unclear whether it will do so, or what the scope would be.

Anti-Kickback Provisions and Requirements

The federal anti-kickback statute prohibits, among other things, knowingly and willfully offering, paying, soliciting or receiving remuneration to induce or in return for purchasing, leasing, ordering or arranging for the purchase, lease or order of any healthcare item or service reimbursable under Medicare, Medicaid or other federally financed healthcare programs. This statute has been interpreted to apply to arrangements between pharmaceutical manufacturers on the one hand and prescribers, purchasers and formulary managers on the other. Violations of the anti-kickback statute are punishable by imprisonment, criminal fines, civil monetary penalties and exclusion from participation in federal healthcare programs. Although there are a number of statutory exemptions and regulatory safe harbors protecting certain common activities from prosecution or other regulatory sanctions, the exemptions and safe harbors are drawn narrowly, and practices that involve remuneration intended to induce prescribing, purchases or recommendations may be subject to scrutiny if they do not qualify for an exemption or safe harbor.

Federal false claims laws prohibit any person from knowingly presenting, or causing to be presented, a false claim for payment to the federal government, or knowingly making, or causing to be made, a false statement to have a false claim paid. Pharmaceutical and other healthcare companies have been prosecuted under these laws for allegedly inflating drug prices they report to pricing services, which in turn were used by the government to set Medicare and Medicaid reimbursement rates, and for allegedly providing free product to customers with the expectation that the customers would bill federal programs for the product. In addition, certain marketing practices, including off-label promotion, have also been alleged by government agencies to violate false claims laws. The majority of states also have statutes or regulations similar to the federal anti-kickback law and false claims laws, which apply to items and services reimbursed under Medicaid and other state programs, or, in several states, apply regardless of the payor.

Coverage, Pricing and Reimbursement

The containment of healthcare costs has become a priority of federal, state and foreign governments, and the prices of drugs have been a focus in this effort. Third party payors are also increasingly challenging the prices charged for medical products and services and examining the medical necessity and cost-effectiveness of medical products and services, in addition to their safety and efficacy. If these third party payors do not consider a product to be cost- effective compared to other available therapies, they may not cover the product after approval as a benefit under their plans or, if they do, the level of payment may not be sufficient to allow a company to sell its products at a profit. The U.S. government, state legislatures and foreign governments have shown significant interest in implementing cost containment programs to limit the growth of government-paid health care costs, including price controls, restrictions on reimbursement and requirements for substitution of generic products for branded prescription drugs. Adoption of such controls and measures, and tightening of restrictive policies in jurisdictions with existing controls and measures, could limit payments for pharmaceuticals.

As a result, the marketability of any product which receives regulatory approval for commercial sale may suffer if the government and third party payors fail to provide adequate coverage and reimbursement. In addition, an increasing emphasis on managed care in the United States has increased and will continue to increase the pressure on drug pricing. Coverage policies, third party

reimbursement rates and drug pricing regulation may change at any time. In particular, the Patient Protection and Affordable Care Act contains provisions that may reduce the profitability of drug products, including, for example, increased rebates for drugs sold to Medicaid programs, extension of Medicaid rebates to Medicaid managed care plans, mandatory discounts for certain Medicare Part D beneficiaries and annual fees based on pharmaceutical companies' share of sales to federal health care programs. Even if favorable coverage and reimbursement status is attained for one or more products that receive regulatory approval, less favorable coverage policies and reimbursement rates may be implemented in the future.

Regulation in the European Union

Product development, the regulatory approval process, and safety monitoring of medicinal products and their manufacturers in the European Union proceed in much the same manner as they do in the United States. Therefore, many of the issues discussed above apply similarly in the context of the European Union. In addition, drugs are subject to the extensive price and reimbursement regulations of the various EU member states.

Clinical trials

As is the case in the United States, the various phases of preclinical and clinical research in the European Union are subject to significant regulatory controls. The Clinical Trials Directive 2001/20/EC, as amended, provides a system for the approval of clinical trials in the European Union via implementation through national legislation of the member states. Under this system, approval must be obtained from the competent national authority of 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 clinical trial application, which must be supported by an investigational medicinal product dossier with supporting information prescribed by the Clinical Trials Directive and corresponding national laws of the member states and further detailed in applicable guidance documents. The sponsor of a clinical trial, or its legal representative, must be based in the European Economic Area. European regulators and ethics committees also require the submission of adverse event reports during a study and a copy of the final study report.

Marketing approval

Marketing approvals under the European Union regulatory system may be obtained through a centralized or decentralized procedure. The centralized procedure results in the grant of a single marketing authorization that is valid for all—currently 28—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, 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. The CHMP also has the discretion to permit other products to use the centralized procedure if it considers them sufficiently innovative or they contain a new active substance. Given our focus on gene therapies, which fall within the category of advanced therapy medicinal products, or ATMPs, and orphan indications, our products and product candidates should typically qualify for the centralized procedure.

In the marketing authorization application, or MAA, the applicant has to properly and sufficiently demonstrate the quality, safety and efficacy of the drug. Under the centralized approval procedure, the CHMP, is responsible for drawing up the opinion of the EMA on any matter concerning the

admissibility of the files submitted in accordance with the centralized procedure, such as an opinion on the granting, variation, suspension or revocation of a marketing authorization, and pharmacovigilance. For ATMPs, the CAT is responsible in conjunction with the CHMP for the evaluation of ATMPs. The CAT is primarily responsible for the scientific evaluation of ATMPs and prepares a draft opinion on the quality, safety and efficacy of each ATMP for which a MAA is submitted. The CAT's opinion is then taken into account by the CHMP when giving its final recommendation regarding the authorization of a product in view of the balance of benefits and risks identified. Although the CAT's draft opinion is submitted to the CHMP for final approval, the CHMP may depart from the draft opinion, if it provides detailed scientific justification.

The CHMP and CAT are also responsible for providing guidelines on ATMPs and have published numerous guidelines, including specific guidelines on gene therapies. These guidelines provide additional guidance on the factors that the EMA will consider in relation to the development and evaluation of ATMPs and include, among other things, the preclinical studies required to characterize ATMPs; the manufacturing and control information that should be submitted in a MAA; and post-approval measures required to monitor patients and evaluate the long term efficacy and potential adverse reactions of ATMPs. Although these guidelines are not legally binding, we believe that our compliance with them is likely necessary to gain and maintain approval for any of our product candidates.

The maximum timeframe for the evaluation of an MAA by the CHMP under the centralized procedure is 210 days after receipt of a valid application. This period will be suspended until such time as the supplementary information requested by the CHMP, or in the case of ATMPs information also requested by the CAT, has been provided by the applicant. Likewise, this time-limit will be suspended for the time allowed for the applicant to prepare oral or written explanations. When an application is submitted for a marketing authorization in respect of a drug which is of major interest from the point of view of public health and in particular from the viewpoint of therapeutic innovation, the applicant may request an accelerated assessment procedure. If the CHMP accepts such request, the time-limit of 210 days will be reduced to 150 days but it is possible that the CHMP can revert to the standard time-limit for the centralized procedure if it considers that it is no longer appropriate to conduct an accelerated assessment.

If the CHMP concludes that the quality, safety and efficacy of the product is sufficiently proven, it adopts a positive opinion. This is sent to the European Commission which drafts a decision. After consulting with the member states, the European Commission adopts a decision and grants a marketing authorization, which is valid for the whole of the European Union.

The European Commission may grant a so-called "marketing authorization under exceptional circumstances". Such authorization is intended for products for which the applicant can demonstrate that it is unable to provide comprehensive data on the efficacy and safety under normal conditions of use, because the indications for which the product in question is intended are encountered so rarely that the applicant cannot reasonably be expected to provide comprehensive evidence, or in the present state of scientific knowledge, comprehensive information cannot be provided, or it would be contrary to generally accepted principles of medical ethics to collect such information. Consequently, marketing authorization under exceptional circumstances may be granted subject to certain specific obligations, which may include the following:

- the applicant must complete an identified programme of studies within a time period specified by the competent authority, the results of which form the basis of a reassessment of the benefit/risk profile;

- the medicinal product in question may be supplied on medical prescription only and may in certain cases be administered only under strict medical supervision, possibly in a hospital and in the case of a radio- pharmaceutical, by an authorised person; and

- the package leaflet and any medical information must draw the attention of the medical practitioner to the fact that the particulars available concerning the medicinal product in question are as yet inadequate in certain specified respects.

A marketing authorization under exceptional circumstances is subject to annual review to reassess the risk-benefit balance in an annual re- assessment procedure. Continuation of the authorization is linked to the annual reassessment and a negative assessment could potentially result in the marketing authorization being suspended or revoked. The renewal of a marketing authorization of a medicinal product under exceptional circumstances, however, follows the same rules as a "normal" marketing authorization. Thus, a marketing authorization under exceptional circumstances is granted for an initial 5 years, after which the authorization will become valid indefinitely, unless the EMA decides that safety grounds merit one additional five-year renewal.

The European Union also provides for a system of regulatory data and market exclusivity. According to Article 14(11) of Regulation (EC) No 726/2004, as amended, and Article 10(1) of Directive 2001/83/EC, as amended, upon receiving marketing authorization, new chemical entities approved on the basis of complete independent data package benefit from eight years of data exclusivity and an additional two years of market exclusivity. Data exclusivity prevents regulatory authorities in the European Union from referencing the innovator's data to assess a generic (abbreviated) application. During the additional two-year period of market exclusivity, a generic marketing authorization can be submitted, and the innovator's data may be referenced, but no generic medicinal product can be marketed until the expiration of the market exclusivity. The overall ten-year period will be extended to a maximum of eleven years if, during the first eight years of those ten years, the marketing authorization holder obtains an authorization for one or more new therapeutic indications which, during the scientific evaluation prior to their authorization, are held to bring a significant clinical benefit in comparison with existing therapies. Even if a compound is considered to be a new chemical entity and the innovator is able to gain the period of data exclusivity, another company nevertheless could also market another version of the drug if such company obtained marketing authorization based on an MAA with a complete independent data package of pharmaceutical test, pre-clinical tests and clinical trials.

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

The obligations of an MAH include:

- *Manufacturing and Batch Release.* MAHs should guarantee that all manufacturing operations comply with relevant laws and regulations, applicable good manufacturing practices, with the product specifications and manufacturing conditions set out in the marketing authorization and that each batch of product is subject to appropriate release formalities.

- *Pharmacovigilance.* MAHs are obliged to establish and maintain a pharmacovigilance system, including a qualified person responsible for oversight, 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 collaboration partner to hold marketing authorizations on our behalf. Any failure by an MAH to comply with these obligations may result in regulatory action against an MAH and ultimately threaten our ability to commercialize our products.

Reimbursement

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

Orphan Drug Regulation

In the European Union, Regulation (EC) No 141/2000, as amended, states that a drug will be designated as an orphan drug if its sponsor can establish:

- that it is intended for the diagnosis, prevention or treatment of a life-threatening or chronically debilitating condition affecting not more than five in ten thousand persons in the Community when the application is made, or that it is intended for the diagnosis, prevention or treatment of a life-threatening, seriously debilitating or serious and chronic condition in the European Union and that without incentives it is unlikely that the marketing of the drug in the European Union would generate sufficient return to justify the necessary investment; and

- that there exists no satisfactory method of diagnosis, prevention or treatment of the condition in question that has been authorized in the European Union or, if such method exists, that the drug will be of significant benefit to those affected by that condition.

Regulation (EC) No 847/2000 sets out further provisions for implementation of the criteria for designation of a drug as an orphan drug. An application for the designation of a drug as an orphan drug must be submitted at any stage of development of the drug before filing of a marketing authorization application.

If 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.

Regulation in Other Countries

For other countries outside of the United States and the European Union the requirements governing the development and approval process as well as post-approval and pricing and reimbursement requirements vary from country to country. In general, clinical studies are to be conducted in accordance with cGCP and the applicable regulatory requirements and the ethical principles originating from the Declaration of Helsinki.

If we fail to comply with applicable foreign regulatory requirements, we may be subject to, among other things, fines, suspension or withdrawal of regulatory approvals, product recalls, seizure of products, operating restrictions and criminal prosecution.

C. Organizational Structure

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

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 2016. We have also leased a facility in Lexington, Massachusetts, where we have begun the build out of a 53,000 square foot manufacturing facility. The lease for this facility terminates in 2024, and subject to the provisions of the lease, may be renewed for two subsequent five year terms. 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—Capital Expenditures" and "—Contractual Obligations and Commitments."

Item 4A *Unresolved Staff Comments*

Not applicable.

Item 5 *Operating and Financial Review and Prospects*

You should read the following discussion and analysis of our financial condition and results of operations in conjunction with the "Selected Consolidated Financial Information" 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

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

Our business was founded in 1998 by scientists who were investigating LPLD at the Academic Medical Center of the University of Amsterdam, or the AMC. In our early years we received funding and subsidized rent from the AMC, government grants, income for cGMP contract manufacturing of biologics for third parties, and small amounts of equity financing. From our first institutional venture capital financing in 2006 until our initial public on February 10, 2014, we funded our operations primarily through private and public placements of equity securities, and other convertible debt securities, in the aggregate amount of €134.8 million ($181.9 million). During this period, we also received total other income, consisting principally of government grants and subsidies, of €5.9 million, and total nonrefundable collaboration funding of €17.0 million. Our predecessor entity, Amsterdam Molecular Therapeutics (AMT) N.V., or AMT, completed an initial public offering of its ordinary shares on Euronext Amsterdam in 2007 and subsequently delisted from that exchange in 2012. We acquired the business of AMT in the first half of 2012.

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

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

As of December 31, 2013, we had cash and cash equivalents of €23.8 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 €26.8 million in fiscal year 2013, €14.7 million in 2012 and €17.3 million in 2011. As of December 31, 2013, we had an accumulated deficit of €144.0 million. We anticipate that our expenses will increase substantially in the future as we:

- complete our EMA-mandated post-approval clinical trial of Glybera and implement an LPLD patient registry;
- conduct a clinical trial of Glybera, either as part of the EMA-mandated post-approval clinical trial or separately, to obtain data needed to file a BLA for Glybera with the FDA;
- seek marketing approval for Glybera in the United States and other countries;

- initiate a Phase I/II clinical trial of AMT-060 for hemophilia B in collaboration with Chiesi;
- advance the preclinical and clinical development of our other product candidates, most of which are at relatively early stages of development, and seek to discover and develop additional product candidates;
- seek marketing approval for any product candidates that successfully complete clinical trials;
- establish a sales, marketing and medical affairs infrastructure in the United States;
- complete the building out and equipping of our manufacturing facility in Lexington, Massachusetts to expand our manufacturing capabilities for Glybera and our pipeline of product candidates;
- fund the ongoing operations of our Lexington facility;
- fund expenses in connection with our new collaboration with 4D Molecular Therapeutics;
- maintain, expand and protect our intellectual property portfolio, including in-licensing additional intellectual property rights from third parties;
- hire additional personnel, particularly in our manufacturing, research, clinical development, medical affairs, commercial and quality control groups;
- add operational, financial and management information systems and related finance and compliance personnel; and
- operate as a public company.

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.

Accounting for our Corporate Reorganization and Strategic Restructuring

At the end of 2011, following the initial rejection of the application for marketing approval for Glybera in the European Union, AMT initiated a strategic restructuring in order to reduce its cost base, conserve resources and improve its financial position. As part of this effort, AMT significantly reduced personnel, programs and expenditures. As a result, we lost a number of employees, including employees with an extensive understanding of our clinical programs as well as our regulatory and financial affairs. AMT implemented a strategic restructuring in the fourth quarter of 2011, as a result of which total staff was reduced from 92 to 49. AMT incurred significant restructuring expenses in connection with this reduction in staff, which were recorded in full during the fourth quarter of 2011. Since that time, we have hired a number of new staff. As of December 31, 2013, we had a total of 87 employees and engaged 33 consultants and contract workers.

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

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

operations. Following the corporate reorganization described above, we restated the financial information of AMT as of and for the year ended December 31, 2011 on a going concern basis.

Collaboration and License Agreements

Chiesi Agreements

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

Glybera agreement

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

We will receive payments for the quantities of Glybera we manufacture and supply to Chiesi, payable in part upon order and in part upon delivery of such product quantities. We will bear the cost of goods sold for the Glybera we deliver, including the royalties and related payments to third parties we must make under the license agreements covering various aspects of the technology underlying the composition and manufacture of Glybera. We estimate that the amount we will retain, net of cost of goods sold, including such third party royalties and related amounts, will be between 20% and 30% of the revenues from sales of Glybera by Chiesi, varying by country of sale. We believe that the amount that we will retain from net sales of Glybera in the European Union will initially be at the lower end of this range and will increase toward the higher end of that range beginning in 2015, upon the expiration of an in-licensed patent on which we pay royalties. In addition, we are required to pay 20% of the gross amount we receive from Chiesi in respect of Glybera product sales to the Dutch government, in repayment of a technical development loan in the outstanding amount of €5.5 million as of December 31, 2013, until the earlier of repayment in full of such amount and 2017, as described below.

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.

4D Molecular Therapeutics

In January 2014, we entered into a collaboration and license agreement with 4D for the discovery and optimization of next-generation AAV vectors. Under this agreement, we have an exclusive license to 4D's existing and certain future know-how and other intellectual property for the delivery of AAV vectors to CNS or liver cells for the diagnosis, treatment, palliation or prevention of all diseases or medical conditions. Under this collaboration, the 4D team, including Dr. David Schaffer, 4D's co-founder and Professor of Chemical and Biomolecular Engineering at the University of California, Berkeley, will establish a laboratory, which we will fund, at a cost of approximately $3.0 million in aggregate over the next three years, to identify next generation AAV vectors. We are also required to make payments for pre-clinical, clinical and regulatory milestones under the collaboration as well as to pay single-digit royalties. In addition, we have granted options to purchase an aggregate of 609,744 ordinary shares in connection with this collaboration, and will recognize resulting share-based payment expense over the next three years. To the extent that the collaboration is successful, we may also incur additional third party costs in developing any product candidates and also in preparing, filing and prosecuting additional patent applications.

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. See "Information on the Company—Intellectual Property—Licenses."

Financial Operations Overview

Revenues

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

During the year ended December 31, 2013, we recognized collaboration revenues of €2.5 million in respect of development activities that were reimbursable by Chiesi under our co-development agreement for hemophilia B. We expect to continue to recognize such collaboration revenues going forward, in accordance with our contractual agreements.

During the year ended December 31, 2013, we also recognized license revenues of €0.4 million. This amount reflects the amortization during the period of the non-refundable upfront payments we received from Chiesi under our collaboration agreements. The balance of €16.6 million of these license revenues will be recognized on a straight-line basis through the remaining period of the intellectual property protection of our manufacturing technologies, which is currently expected to be until September 2032.

The timing of our operating cash flows may vary from the recognition of the related amounts, as we defer the recognition of some upfront payments, including the upfront payments under our Chiesi agreements, and recognize these as revenue when earned or over a defined period, while we treat other revenue, such as milestone payments or service fees, as earned when received. We expect our revenues

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

Other Income

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

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

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

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

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

Cost of Goods Sold

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

We also include in cost of goods sold amounts that we are required to repay to the Dutch government in respect of a technical development loan that we received in the period from 2000 to 2005 to support the early development of Glybera. As of December 31, 2013, the total amount of

principal and interest outstanding was €5.5 million. Under the terms of this contingent commitment, we are required to make repayments based on the timing and amount of revenues we receive from product sales of Glybera. In connection with our receipt of upfront payments from Chiesi for the commercialization of Glybera, we repaid €0.8 million of this loan in September 2013, which we recorded as cost of goods sold although no product sales occurred. No further payments will be made until sales of Glybera commence. We expect to pay to the Dutch government 20% of any gross amounts we receive from Chiesi in connection with sales of Glybera, as and when received, until the earlier of such time as the loan is repaid in full or December 31, 2017. Amounts that remain outstanding as of December 31, 2017, if any, would be forgiven. We have not recorded any liability for these amounts. To the extent we generate revenues from the sale of Glybera, we will recognize a liability and a corresponding charge to cost of goods sold in future periods.

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

Research and Development Expenses

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

During the period from 2006, when we received our first significant venture capital equity investment, to December 31, 2013, we incurred an aggregate of €96.7 million in research and development expenses. Our total research and development expenses in 2013 were €13.2 million. In addition, we began to capitalize our development expenses related to Glybera from March 21, 2013. We capitalized €3.1 million of such expenses in fiscal year 2013, which we expect to begin amortizing once sales of Glybera commence, over the period through September 2032. We allocate our direct research and development expenses to our various programs on the basis of actual external expenses incurred in respect of each program and our allocation of time spent by our research and development team on each program. We do not allocate our overhead expenses to specific development programs. Our research and development expenses mainly relate to the following key programs:

- *Glybera.* We are undertaking preparations for the EMA-mandated post-approval clinical trial and patient registry. In addition, we are undertaking preparations for the submission of an IND with the FDA in the first half of 2014. We bear all of the costs of this program outside of the territories covered by the Chiesi agreement. Certain costs, including the patient registry for territories covered by the Chiesi agreement, will be shared equally with Chiesi.

- *Hemophilia B.* We plan to initiate a Phase I/II clinical trial of AMT-060 for the treatment of hemophilia B in the second half of 2014 in collaboration with Chiesi. Under our co-development agreement, we and Chiesi will each bear half of the development costs of this program.

- *Acute intermittent porphyria (AIP).* We have incurred costs related to the development and manufacture of clinical supplies of AMT-021 for the treatment of AIP provided to our collaboration partner, Digna Biotech, for its ongoing Phase I clinical trial in this indication.

- *CNS programs.* We have incurred costs related to the development and manufacture of clinical supplies of AMT-110 for the treatment of Sanfilippo B provided to our collaboration partner, Institut Pasteur, for its ongoing Phase I/II clinical trial. We also incur expenses related to the research and preclinical activities related to our other CNS programs.

- *Technology platform development and other research.* We incur significant research and development costs related to our gene delivery and manufacturing technology platform that are

applicable across all of our programs, as well as our other research programs, including intellectual property expenses, depreciation expenses and facility costs. These costs are not allocated to specific projects.

The table below sets forth our direct research and development expenses by program for the years ended December 31, 2011, 2012 and 2013.

(€ in thousands, except percentages)	YEAR ENDED DECEMBER 31, 2011	2012	CHANGE %	2013	CHANGE %
Glybera program*	4,381	1,055	(76)%	2,727	158%
Hemophilia B program	671	1,131	69%	3,034	168%
AIP program	1,383	1,055	(24)%	241	(77)%
CNS programs	363	922	154%	822	(11)%
Technology platform development and research programs	8,702	6,068	(30)%	6,358	5%
Total	15,500	10,231	(34)%	13,182	29%

* Excludes capitalized development expenses of €3.1 million in 2013 (2011 and 2012: nil).

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

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

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

We have incurred significant expenses in the development of Glybera. Under applicable accounting principles, we capitalize development expenses upon receipt of marketing approval for a product

candidate, provided that we have the technical, scientific and financial resources to complete the development and commercialization of the program. We received marketing approval from the European Commission for Glybera for a subset of LPLD patients in October 2012. Because we did not have sufficient financial resources at that time to complete the development of Glybera, including the post-approval activities required by the EMA prior to commercial launch, we did not capitalize the development expenses related to Glybera during the year ended December 31, 2012. Following our receipt of an additional €10.0 million in convertible debt financing in the first quarter of 2013, we determined that we had sufficient financial resources to complete these post-approval activities, and accordingly began to capitalize the related development expenses in the first quarter of 2013.

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

In addition, in connection with the collaboration and license agreement we entered into with 4D Molecular Therapeutics during January 2014, we will incur additional expenses as we fund a joint research effort with 4D. Further, we granted options to purchase an aggregate of 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 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. See "Information on the Company—Strategic Collaboration: 4D Molecular Therapeutics."

Selling, General and Administrative Expenses

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

Other Losses—Net

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

Finance Income

Our finance income consists of interest income earned on our cash and cash equivalents and gains on our derivative instruments, described below. We deposit our cash and cash equivalents primarily in

savings and deposit accounts with original maturities of three months or less. Savings and deposit accounts have historically generated only minimal interest income.

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

Finance Expense

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

A. Operating Results

Overview

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

Comparison of the year ended December 31, 2011, 2012 and 2013

	YEAR ENDED DECEMBER 31,		
(€ in thousands)	**2011**	**2012**	**2013**
Revenues:			
License revenues	—	—	440
Collaboration revenues	—	—	2,503
Total revenues	—	—	2,943
Cost of goods sold	—	—	(800)
Other income	2,192	649	585
Expenses:			
Research and development expenses	(15,500)	(10,231)	(13,182)
Selling, general and administrative expenses	(3,807)	(4,564)	(11,628)
Other losses, net	(26)	(45)	(453)
Operating result	(17,141)	(14,191)	(22,535)
Finance income	277	22	102
Finance expense	(436)	(547)	(4,387)
Net loss	(17,300)	(14,716)	(26,820)

Revenues

License revenues of €0.4 million in the year ended December 31, 2013 related to the amortization of the upfront payment received from Chiesi in July 2013.

Collaboration revenues of €2.5 million in the year ended December 31, 2013 consisted mainly of reimbursements of covered expenses by Chiesi under our agreements (€2.2 million), together with revenue from Institut Pasteur relating to our Sanfilippo B collaboration (€0.3 million). We had no revenues in the years ended December 31, 2012 or 2011.

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 years ended the year ended December 31, 2012 or 2011.

Other Income

Other income for the year ended December 31, 2013 was €0.6 million, was in line with the €0.6 million recognized for the year ended December 31, 2012. This income represented reimbursement of payroll taxes received from the Dutch government and the receipt of grants to support research projects.

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

Research and Development Expenses

Research and development expenses for the year ended December 31, 2013 were €13.2 million, a 29% increase from the €10.2 million incurred for the twelve months ended December 31, 2012. This increase reflected the expansion of our research and development activities to support the further development of our pipeline product candidates as well as expenditure during the first quarter of 2013 on development to support the planned commercial launch of Glybera in the European Union (from March 21, 2013 onwards Glybera development expenditure in the European Union has been capitalized). Following our receipt of additional convertible loan and debt funding in the first nine months of 2013, we increased the level of research and development expenditures compared with the relatively low level of expenditure during 2012 attributable to our strategic restructuring at the end of 2011.

Glybera-related raw materials that cannot be used for commercial purposes since March 2013 are capitalized as development costs (prior to March 2013 they were 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.

Research and development expenses for the year ended December 31, 2012 were €10.2 million, a 34% decrease from the €15.5 million incurred for the year ended December 31, 2011. The decrease reflected the strategic restructuring and related reduction in our workforce we undertook at the end of

2011. Following the reduction in staff, we also reduced our overall level of activity. Furthermore, during the first half of 2012, we focused on our early-stage programs, which generally require less investment than more advanced programs.

Selling, General and Administrative Expenses

Selling, general and administrative expenses for the year ended December 31, 2013 were €11.6 million, a 155% increase from the €4.6 million incurred for the year ended December 31, 2012. This increase resulted principally from our increased headcount in 2013 as we continued to ramp up our operations following our strategic restructuring at the end of 2011, share-based expenses relating to the costs of warrants and options granted during the period, and increased commercial, legal and other advisory fees.

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

Other losses—Net

Other losses—net for the year ended December 31, 2013 were a loss of €0.5 million; a 907% increase from the loss of €0.05 million for the year ended December 31, 2012, and related to foreign exchange impacts. This increase reflects changes in the exchange rate between the euro and the U.S. dollar.

Other losses—net were not material in 2011.

Finance Income

Finance income for the year ended December 31, 2013 was €0.1 million, a 364% increase from the €0.02 million for the year ended December 31, 2012, and a 92% decrease from the €0.3 million for the year ended December 31, 2011. This reflects our average cash balances and low interest rates in both periods.

Finance Expense

Finance expense for the year ended December 31, 2013 was €4.4 million, compared with €0.5 million for the year ended December 31, 2012. This increase primarily related to the revaluation and/or early conversion of the embedded derivatives related to our convertible loans and the venture loan, which totaled €3.5 million during the year ended December 31, 2013.

Finance expense remained relatively stable at €0.5 million for the year ended December 31, 2012 compared with €0.4 million for the year ended December 31, 2011, principally representing interest due on convertible loans in 2011, and the charge on the movement in the value of the derivative element of our convertible loans, which were converted on our restructuring in April 2012.

B. 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 on February 10, 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 €134.8 million ($181.9 million). During this period, we also received total other income, consisting principally of

government grants and subsidies, of €5.9 million, and total nonrefundable collaboration funding of €17.0 million, and $10.0 million (€7.5 million) in venture debt financing.

We had a net loss of €26.8 million in the year ended December 31, 2013, €14.7 million in full year 2012 and €17.3 million in full year 2011. As of December 31, 2013, we had an accumulated deficit of €144.0 million. 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.

Cash flows

Our cash and cash equivalents as of December 31, 2013 were €23.8 million. The table below summarizes our consolidated cash flow data for the years ended December 31, 2011, 2012 and 2013:

	YEAR ENDED DECEMBER 31,		
(€ in thousands)	**2011**	**2012**	**2013**
Net cash used in operating activities	(16,705)	(11,277)	(4,136)
Net cash used in investing activities	(162)	(832)	(5,971)
Net cash generated from financing activities	108	11,272	33,642

Net Cash Used in Operating Activities

Net cash used in operating activities was €4.1 million in the year ended December 31, 2013, a 63% decrease from net cash used in operating activities of €11.3 million in the year ended December 31, 2012. The change reflected the receipt of the upfront payment under our collaboration agreements with Chiesi, for a total of €17.0 million.

Net cash used in operating activities was €11.3 million in 2012, a 33% decrease from €16.7 million in 2011. The decrease reflected the reduction in net loss before corporate income tax for 2012 compared to 2011, which in turn was due to the strategic restructuring and related reduction in our workforce we undertook at the end of 2011. Following the reduction in staff we also reduced our overall activity. In 2012 our net loss before corporate income tax was €14.7 million, a decrease of €2.6 million compared to 2011. In addition, changes in overall composition of our working capital balance also resulted in an overall reduction in the cash used in operations.

Net Cash Used in Investing Activities

Net cash used in investing activities was €6.0 million in the year ended December 31, 2013, compared with net cash used in investing activities of €0.8 million in the year ended December 31, 2012. The increase reflected the capitalization of €3.1 million of Glybera development expenses beginning in March 2013, as well as the capitalization of payments due to licensors following the upfront payment under our collaboration agreements with Chiesi.

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

Net Cash Generated from Financing Activities

Net cash generated from financing activities was €33.6 million in the year ended December 31, 2013, compared with net cash generated from financing activities of €11.3 million in the year ended December 31, 2012. The increase reflected the receipt of €12.0 million in funding from the issuances of convertible notes (all of which were fully converted in the period), $10.0 million in funding from a

venture loan and the receipt of the €14.0 million equity investment from Chiesi during year ended December 31, 2013.

Net cash generated from financing activities was €11.3 million in year ended December 31, 2012, compared with €0.1 million in year ended December 31, 2011. The increase reflected our private placements of convertible notes and equity securities in year ended December 31, 2012 in connection with and following our corporate reorganization.

Cash and Funding Sources

The table below summarizes our sources of financing for the years ended December 31, 2011, 2012 and 2013:

(€ in thousands)	EQUITY CAPITAL(1)	CONVERTIBLE NOTES	OTHER DEBT	TOTAL
Year ended December 31, 2013	14,294	11,999	7,492	33,785
Year ended December 31, 2012	9,774	1,498	—	11,272
Year ended December 31, 2011	108	—	—	108
Total	24,176	13,497	7,492	45,165

(1) Excludes shares issued upon conversion of convertible notes.

Our sources of financing in the year ended December 31, 2013 were:

- the issuance and sale of 90,747 ordinary shares to our employees for gross proceeds of €0.3 million;
- the issuance and sale of €12.0 million of our convertible notes;
- a venture loan in the principal amount of $10.0 million from Hercules Technology Growth Capital, or Hercules, pursuant to a loan and security agreement dated June 14, 2013, or the Hercules Agreement; and
- the acquisition of 1,109,214 ordinary shares by Chiesi for €14.0 million.

As of December 31, 2013, we had debt of $10 million, equivalent to €7.5 million, which consisted solely of amounts outstanding under the Hercules Agreement.

Funding Requirements

We believe our cash and cash equivalents, including proceeds of our initial public offering in February 2014, will enable us to fund our operating expenses, including our debt repayment obligations as they become due, and capital expenditure requirements, including the build-out of our Lexington, Massachusetts facility, for at least the next 12 months. We have based this estimate on assumptions that may prove to be incorrect, and we could use our capital resources earlier than we currently expect. Our future capital requirements will depend on many factors, including:

- the commercial success of Glybera, including the timing and amount of revenues generated, as well as our cost of goods sold;
- our collaboration agreements remaining in effect and our ability to obtain research and development funding and achieve milestones under these agreements;
- the progress and results of our current and planned clinical trials, including for Glybera, and those of our collaborators;

- the scope, progress, results and costs of preclinical development, laboratory testing and clinical trials for our additional product candidates;
- the number and development requirements of other product candidates that we pursue;
- the cost, timing and outcome of regulatory review of our product candidates, particularly for approval of Glybera in the United States;
- the cost and timing of future commercialization activities by us or our collaborators, including product manufacturing, marketing, sales and distribution, for Glybera and any of our product candidates for which we receive marketing approval in the future;
- the amount and timing of revenue, if any, we receive from commercial sales of any product candidates for which we receive marketing approval in the future;
- expenses in connection with our collaboration with 4D Molecular Therapeutics;
- the costs and timing of preparing, filing and prosecuting patent applications, maintaining and enforcing our intellectual property rights and defending any intellectual property-related claims;
- the extent to which we acquire or in-license other products or technologies; and
- the cost and progress of the build-out of our Lexington, Massachusetts manufacturing facility.

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

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

Capital Expenditures

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

	YEAR ENDED DECEMBER 31,		
(€ in thousands)	**2011**	**2012**	**2013**
Investments in property, plant and equipment	200	392	1,336
Investments in intangible assets	109	553	4,652
Total	309	945	5,988

We are currently building out a 53,000 square foot leased manufacturing facility in Lexington, Massachusetts. We anticipate that the total construction costs will amount to approximately $16.4 million (€ 11.9 million), of which the landlord is obligated to pay $7.3 million (€5.3 million) in landlord improvements. In addition, we anticipate the total investment in property, plant and equipment to be approximately $6.4 million (€4.7 million). As of December 31, 2013, we had capitalized $1.8 million (€1.3 million) and had contractual commitments of a further $14.9 million (€10.8 million). In addition, we provided a landlord deposit of $1.2 million (€0.9 million). We anticipate that we will have paid the full amount of these build-out costs by the end of the second quarter of 2014.

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

Hercules Loan and Security Agreement

We are party to a Loan and Security Agreement entered into with Hercules on June 13, 2013. Under the Loan and Security Agreement, we borrowed $10.0 million (€7.5 million) from Hercules, bearing interest at a variable rate of the greater of 11.85% or an amount equal to 11.85% plus the prime rate of interest minus 3.25%. We are required to pay only interest in monthly payments until October 2014. From October 2014, we will be required to make monthly payments of interest and principal in the amount of $387,000 (€281,000). The loan matures on October 1, 2016, when we will be required to make a final payment of $2.6 million (€1.9 million). The loan and security agreement also provides for payment of a maturity charge, the amount of which was reduced in exchange for the issuance to Hercules, on September 24, 2013, of 37,174 warrants, at an exercise price of $13.45 per share.

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

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

As of December 31, 2012, we had neither significant long-term interest-bearing assets nor significant long-term interest bearing liabilities other than our convertible notes, which were subsequently converted into ordinary shares on July 26, 2013. 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%.

Credit Risk

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

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

Liquidity Risk

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

Internal Control Over Financial Reporting

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

- a lack of accounting resources required to fulfill IFRS and SEC reporting requirements,
- a lack of comprehensive IFRS accounting policies and financial reporting procedures; and
- a lack of segregation of duties given the size of our finance and accounting team.

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

Neither we nor our independent registered public accounting firm undertook a comprehensive assessment of our internal control for purposes of identifying and reporting material weaknesses, significant deficiencies and control deficiencies in our internal control over financial reporting as we will be required to do now that, effective February 10, 2014, we are a public company. We believe it is possible that, had we performed a formal assessment of our internal control over financial reporting or had our independent registered public accounting firm performed an audit of our internal control over financial reporting, additional control deficiencies may have been identified.

We have progressed the evaluation of our internal control over financial reporting. We have also taken several remedial actions to address the material weaknesses that have been identified. To this end, we have hired additional staff for the finance department who have external reporting and IFRS

experience, and experience with establishing appropriate financial reporting policies. Moreover, we have engaged a team of external consultants to assist us to improve our corporate governance and internal control procedures and help us design and implement a structured control environment for complying with the Sarbanes-Oxley Act of 2002, and we have devoted significant efforts to remedy any deficiencies or control gaps identified in the process. We expect to complete the measures above as soon as practicable and we will continue to implement measures to remedy our internal control deficiencies in order to meet the deadline imposed under Section 404 of the Sarbanes-Oxley Act. However, the implementation of these measures may not fully address the existing material weaknesses in our internal control over financial reporting, and we cannot yet conclude that they have been fully remedied.

The process of designing and implementing an effective financial reporting system is a continuous effort that requires us to anticipate and react to changes in our business and the economic and regulatory environments and to expend significant resources to maintain a financial reporting system that is adequate to satisfy our reporting obligations. See "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."

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 2011 or 2012.

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

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

Where such arrangements can be divided into separate units of accounting (each unit constituting a separate earnings process), the arrangement consideration is allocated to the different units based on their relative fair values and recognized over the respective performance period. Where the arrangement cannot be divided into separate units, the individual deliverables are combined as a single unit of accounting and the total arrangement consideration is recognized over the estimated

collaboration period. This analysis requires considerable estimates and judgments to be made by us, including estimates of the relative fair values of the various elements included in such agreements and the estimated length of the respective performance periods.

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

Research and Development Expenses

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

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

The financial terms of these agreements are subject to negotiation, vary from contract to contract and may result in uneven payment flows. There may be instances in which payments made to vendors and other counterparties will exceed the level of services provided and result in a prepayment of the research and development expenses. In accruing service fees, we estimate the time period over which

services will be performed and the level of effort to be expended in each period. If the actual timing of the performance of services or the level of effort varies from our estimate, we adjust the accrual or prepayment expense accordingly. Although we do not expect our estimates to be materially different from amounts actually incurred, our understanding of the status and timing of services performed relative to the actual status and timing of services performed may vary and could result in reporting amounts that are too high or too low in any particular period.

Corporate and Other Taxes

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

We did not recognize any taxes or income during the periods covered by financial statements contained in this annual report, since we are in a loss making position and have a history of losses. As of December 31, 2013, the total amount of tax losses carried forward was €130.9 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.

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 years ended December 31, 2012 and 2013 we have reviewed the carrying amount of these assets and determined that no adjustments to carrying values were required.

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

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

We have determined that no impairment was required to be recorded during the years ended December 31, 2012 or 2013. 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.

This conclusion was further supported by the IPO proceeds realized in February 2014 and the current market capitalization.

Based on our expectations of revenues and gross margin from anticipated sales of Glybera by Chiesi, we have determined that no impairment charge in respect of intangible assets relating to Glybera is necessary. These expectations are based principally on our estimate of the market size for Glybera and the gross margin that we expect to realize.

Compound Financial Instruments

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

Share-Based Compensation

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

- the expected life of the option award, which we 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.

Prior to our acquisition of the AMT business on April 5, 2012, AMT was listed on Euronext Amsterdam from June 2007 through April 2012. This period provided company-specific historical and implied volatility information. Since the de-listing of AMT in April 2012, we have not had the same level of company-specific historical and implied volatility information; therefore, we estimate the expected volatility based on the historical volatility of publicly traded peer companies with a similar focus on gene therapies, biological products or orphan diseases, including Oxford Biomedica plc, MolMed S.p.A., Transgene SA, Sarepta Therapeutics, Inc., Sangamo Biosciences Inc. and Synageva BioPharma Corp.

We account for share options as an expense in the statement of comprehensive income over the estimated vesting period, with a corresponding contribution to equity. See Note 12 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 April 11, 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:

GRANT DATE	NUMBER OF ORDINARY SHARES UNDERLYING OPTIONS GRANTED	EXERCISE PRICE PER ORDINARY SHARE	ESTIMATED FAIR VALUE PER ORDINARY SHARE AT GRANT DATE	RETROSPECTIVE FAIR VALUE PER ORDINARY SHARE AS OF GRANT DATE[1]	ESTIMATED FAIR VALUE PER OPTION AS OF GRANT DATE
April 5, 2012	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

(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.

Of the 2,301,588 options which have been granted under our equity incentive plans and in connection with our agreement with 4D Molecular Therapeutics, and that remained outstanding as of April 11, 2014, an aggregate of 478,217 options were granted to members of the management board. 1,507,443 options which have been granted vested in full on or before the closing of our initial public offering on February 10, 2014, which will result in the acceleration of any unrecognized expense related to these options. As of December 31, 2013, the unrecognized expense related to the options which have been granted and remained outstanding was €1.7 million.

The intrinsic value of all outstanding vested and unvested options as of April 11, 2014 was $30.1 million, based on the our initial public offering price of $17.00 per ordinary share (€12.60 per ordinary share) on February 10, 2014, and was based on 2,301,588 ordinary shares issuable upon the exercise of options outstanding as of the date of this annual report with a weighted average exercise price of €2.89 per share.

Recent Accounting Pronouncements

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

A number of new standards and amendments to standards and interpretations (e.g IFRS9, IAS36, IAS39) are effective for annual periods beginning after January 1, 2014 and have not been applied in preparing these consolidated financial statements. None of these are expected to have a material effect on the consolidated financial statements of the Company.

C. Research and Development Expenses, Patents and Licences, 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, 2017, based upon a percentage of revenues which are derived from the sale of Glybera, if any. If future amounts received are not sufficient to repay the grant on or prior to December 31, 2017, or if there are no revenues generated from Glybera, the remaining balance will be forgiven. The amount of this contingent commitment as of December 31, 2013 totaled €5.5 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.

During the periods presented in this annual report, 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, 2013 that are expected to have an impact on liquidity and cash flows in future periods.

	PAYMENTS DUE BY PERIOD				
(€ in thousands)	LESS THAN 1 YEAR	BETWEEN 1 AND 2 YEARS	BETWEEN 2 AND 5 YEARS	MORE THAN 5 YEARS	TOTAL
License maintenance obligations(1)(2)	327	300	687	856	2,170
Debt obligations	1,498	3,372	4,442	—	9,312
Operating lease obligations	1,243	1,766	4,287	7,927	15,223
Finance lease obligations	156	168	134	—	458
Construction commitment US Facility	10,824	—	—	—	10,824
Total	14,048	5,606	9,550	8,783	37,987

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

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

The table above does not include:

- Payments we may be obligated to make under our license or collaboration agreements, other than fixed periodic maintenance costs. Such additional payment obligations may be material. See "—Collaboration and License Agreements" and "Information on the Company—Intellectual Property—Licenses".

- Our obligations to repay the Dutch technical development loan described above.

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

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:

NAME	AGE	POSITION	MEMBER SINCE(1)	TERM EXPIRES
Ferdinand Verdonck	71	Member of the Supervisory Board (Chairman)	2012	2017
Sander Slootweg	45	Member of the Supervisory Board	2012	2015
Sander van Deventer	59	Member of the Supervisory Board	2012	2016
Joseph M. Feczko	65	Member of the Supervisory Board	2012	2016
François Meyer	65	Member of the Supervisory Board	2012	2015
David Schaffer	44	Member of the Supervisory Board	2014	2016
Paula Soteropoulos	46	Member of the Supervisory Board	2013	2017

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

Ferdinand Verdonck has served as our chairman since July 2012 and served as chairman of the AMT supervisory board from April 2007 until July 2012. He is a director on the boards of J.P. Morgan European Investment Trust, Groupe SNEF, Laco Information Services and Virtus Funds. From 1992 to 2003, he was the managing director of Almanij NV, a financial services company which has since merged with KBC, and his responsibilities included company strategy, financial control, supervision of executive management and corporate governance, including board participation in publicly-traded and

privately-held companies in many countries. He served as a member of the board of directors and chairman of the audit committee of two biotechnology companies in Belgium, Movetis and Galapagos. He has previously served as chairman of Banco Urquijo, a director of Dictaphone Corporation and a director of the Dutch Chamber of Commerce for Belgium and Luxembourg, member of the General Council and chairman of the audit committee of the Vlerick Leuven Ghent Management School. Mr. Verdonck holds a law degree from KU Leuven and degrees in economics from KU Leuven and the University of Chicago. We believe that Mr. Verdonck is qualified to serve on our supervisory board due to his expertise in the financial services and manufacturing industries and his service on the boards of directors of other companies.

Sander Slootweg has served as a member of our supervisory board since April 2012 and served as member of the AMT supervisory board from September 2006 to April 2008, including as Chairman from 2006 to 2007. Mr. Slootweg is a managing partner at Forbion Capital Partners, the Netherlands, a venture capital firm he co-founded in 2006. He currently serves on the boards of Forbion's portfolio companies Xention, Ltd, Pulmagen Therapeutics, Ltd, Dezima Pharma, B.V. (Chairman), Ario Pharma Ltd. and Oxyrane, Ltd. In addition, in recent years Mr. Slootweg has served on the boards of Argenta Discovery Ltd (sold to Galapagos in 2010), Alantos Pharmaceuticals, Inc. (sold to Amgen in 2007), BioVex Group, Inc. (sold to Amgen in 2011), Impella Cardiosystems AG (sold to Abiomed, Inc. in 2005), Glycart AG (sold to Roche in 2005), Cambridge Drug Discovery Ltd (sold to Biofocus Plc in 2001), Fovea Pharmaceuticals S.A. (sold to Sanofi-Aventis in 2009) and Pieris AG. Mr. Slootweg holds degrees in Business and Financial Economics from the Free University of Amsterdam and in Business Administration from Nijenrode University, The Netherlands. We believe that Mr. Slootweg is qualified to serve on our supervisory board due to his expertise in the healthcare technology industry and his service on the boards of directors of other companies.

Sander van Deventer has served as a member of our supervisory board since April 2012 and served as member of the AMT supervisory board from April 2010 to April 2012. Dr. van Deventer was one of our co-founders and currently chairs uniQure's Scientific Advisory Board. He served as our interim Chief Executive Officer from 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.

Joseph M. Feczko has served as a member of our supervisory board since April 2012 and served as a member of the AMT supervisory board from August 2010 to April 2012. Dr. Feczko worked for Pfizer Inc. from 1982 to 1992 and from 1996 to 2009, where he held positions of increasing responsibility in clinical research, regulatory affairs and safety culminating in the role of Senior Vice President and Chief Medical Officer. From 1992 to 1996, Dr. Feczko was Medical Director for GlaxoSmithKline R&D in the United Kingdom. Dr. Feczko is chairman of the board of directors at Cardoz Pharmaceuticals AB, and a director of Keryx Biopharmaceuticals, Inc. and ChemoCentryx Inc., as well as a member of the supervisory board of Cytheris. He is also a member of the board of directors of Accordia Global Health Foundation Research!America, and the Foundation of National Institute of Health, and a trustee of the New York Academy of Medicine. Dr. Feczko is a member of

the Technical Expert Committee for the International Trachoma Initiative of the Task Force for Global Health. Between 2006-2011 he was a member of the Governing Board of the Technology Strategy Board of the United Kingdom. Dr. Feczko is Board Certified in Internal Medicine and Infectious Diseases. Dr. Feczko holds a bachelor of science degree from Loyola University and an M.D. from the University of Illinois College of Medicine. We believe that Dr. Feczko is qualified to serve on our supervisory board due to his expertise in the pharmaceutical and biotechnology industries.

François Meyer has served as a member of our supervisory board since April 2012 and served as a member of the AMT supervisory board from July 2010 to April 2012. Dr. Meyer was until recently CEO and Chairman of the board of TxCell SA, a cell therapy company located in France, and of which he is currently Executive Chairman. Prior to this, he was CEO of Gencell, a fully owned gene therapy subsidiary of Aventis until 2006. He was senior vice president R&D at Aventis Pharma until 2002 and prior to that he led global research at Rhone Poulenc Rorer. In the earlier part of his career he held senior management positions at Sandoz and led the gene and cell therapy business. He was a member of the board of directors or the scientific advisory board of a number of biotech companies in the gene and cell therapy area including Introgen Therapeutics, Inc., Gene Therapy Inc., Systemix, Inc. and Biotransplant, Inc. We believe that Dr. Meyer is qualified to serve on our supervisory board due to his expertise and insight in the biotechnology industry.

David Schaffer 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.

Paula Soteropoulos has served as a member of our supervisory board since July 2013. Ms. Soteropoulos is Senior Vice President and General Manager, Cardiometabolic Business and Strategic Alliances at Moderna Therapeutics, Inc., a position she has held since July 2013. Previously, Ms. Soteropoulos has worked at Genzyme Corporation, a biotechnology company, from 1992 to 2013, most recently as Vice President and General Manager, Cardiovascular, Rare Diseases. Ms. Soteropoulos holds a bachelor of science degree in chemical engineering and a master of science degree in chemical and biochemical engineering, both from Tufts University, and holds an executive management certificate from the University of Virginia, Darden Graduate School of Business Administration. We believe Ms. Soteropoulos is qualified to serve on our supervisory board due to her extensive experience in the biotechnology industry.

Management board

The following table sets out information with respect to each of our management board members, their respective ages and their positions at uniQure as of the date of this annual report. The business

address of our management board members is our registered office address at Meibergdreef 61, Amsterdam 1105 BA, the Netherlands.

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

(1) Mr. Morgan has tendered his resignation, which will be effective May 20, 2014.

Jörn Aldag has served as our chief executive officer since he joined AMT, now uniQure, in October 2009. He has led our corporate development including the expansion of our gene therapy pipeline, the marketing authorization process with the EMA for Glybera and the recapitalization of AMT to form uniQure. Before joining our company he was instrumental in building Evotec AG, a drug discovery company listed on the Frankfurt Stock Exchange, serving as chief financial officer from 1997 to 2000 and as president and chief executive officer from 2001 to 2009. Prior to Evotec, Mr. Aldag served in various financial management positions at MAN AG, and as Business Director at Treuhandanstalt, the agency responsible for privatizing the East German economy after the German reunification. Mr. Aldag is Chairman of Molecular Partners AG, Zurich, Switzerland, and holds business degrees from the Harvard Business School (Advanced Management Program) and the European Business School. We believe that Mr. Aldag is qualified to serve on our management board due to his broad expertise in the biotechnology industry and his deep general management experience.

Piers Morgan has served as our chief financial officer since he joined AMT in December 2009. Mr. Morgan is currently chairman of the board and a member of the audit committee of Trino Therapeutics Ltd, a biotechnology company. He has more than 13 years of experience as chief financial officer of several biotechnology companies, including Phytopharm plc, BioAlliance Pharma SA, and Arrow Therapeutics Ltd. Prior to this period, he spent ten years in investment banking, working in mergers & acquisitions and equity capital markets with Close Brothers and Ernst & Young Corporate Finance. He qualified as a chartered accountant in London with PricewaterhouseCoopers. Mr. Morgan holds a degree in law and management studies from Cambridge University. We believe that Mr. Morgan is qualified to serve on our management board due to his expertise in the biotechnology industry and his accounting background. Mr. Morgan has tendered his resignation as our chief financial officer and member of the management board, effective May 20, 2014.

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.

NAME	AGE	POSITION
Philip Astley-Sparke	42	President, U.S. Operations
Christian Meyer, M.D.	46	Chief Medical Officer
Harald Petry	54	Chief Science Officer
Hans Preusting	51	Chief Business Officer
Hans Christian Rohde	56	Chief Commercial Officer

Philip Astley-Sparke has served as the president of our U.S. operations since January 2013. Mr. Astley-Sparke has been a venture partner at Forbion Capital Partners, a venture capital fund, since May 2012. He served as vice president and general manager at Amgen, Inc., a biopharmaceutical company, until December 2011, following Amgen's acquisition of BioVex Group, Inc., a biotechnology company, in March 2011. Mr. Astley-Sparke had been president and chief executive officer of BioVex Group since 2007, which he joined in 2000, and previously served in the roles of President & COO and CFO. He oversaw the company's relocation to the United States where he grew operations from scratch, including overseeing the construction of a commercial-grade manufacturing facility. Prior to Biovex Group, Mr. Astley-Sparke was a healthcare investment banker with Chase H&Q/Robert Fleming. He qualified as a chartered accountant with Arthur Andersen in London and holds a bachelor's degree in cellular pathology and molecular pathology from Bristol University in the United Kingdom. He also serves as chairman of the board of Oxyrane, a biotechnology company.

Christian Meyer, M.D. has served as our chief medical officer since October 2013. Dr. Meyer has more than 13 years of clinical research experience with both biotechnology companies and large pharma, with particular expertise in the development of treatments for rare diseases, including acute intermittent porphyria and lysosomal storage disorders. From 2010-2013 he was the chief medical officer at Cardoz AB, a pharmaceutical company. Prior to that, from 2006 to 2010, Dr. Meyer held leadership positions in clinical development at Symphogen A/S, a biopharmaceutical company, where he was senior vice president for medical affairs and vice president of clinical development. Prior to Symphogen A/S, he played an important role in clinical development at Zymenex A/S and spent five years in clinical development at Novo Nordisk A/S, both biopharmaceutical companies. Dr. Meyer received both his M.D. and Ph.D. degrees from the University of Copenhagen, Denmark.

Harald Petry has served as our chief science officer since January 2012. Dr. Petry joined AMT in May 2007 as director of research and development. He has worked in the area of gene therapy for more than 15 years and has extensive experience in pharmaceutical research. Prior to joining us, he worked at Jenapharm GmbH (Germany), a pharmaceutical company, from 2001 to 2002 and Berlex Biosciences (US), a biotechnology company, from 2002 to 2007 in different functions with increasing managerial and leadership responsibility. Dr. Petry holds his doctoral degree in biology from Justus-Liebig-Universität Giessen.

Hans Preusting has served as our chief business officer since July 2011, including at AMT where he first joined us as a Director of Process Development and Manufacturing in August 2006. He holds a PhD in biochemistry and an MBA from Rotterdam School of Management. He has more than 20 years of experience in product development and manufacturing using fermentation and cell culture techniques. Prior to joining us, he was at Solvay Pharmaceuticals, DSM and Gist-brocades. Dr. Preusting holds two patents and has published more than 20 scientific articles.

Hans Christian Rohde has served as our chief commercial officer since December 2012. Mr. Rohde has almost 25 years of experience in commercial roles at leading biotechnology and pharmaceutical companies. From 2007 until 2012 he was chief commercial officer at Basilea Pharmaceutica, a pharmaceutical company, and a member of its executive management committee with responsibility for global commercial operations, marketing, supply chain, medical affairs, pricing and market access. Prior to Basilea Pharmaceutica, Mr. Rohde was corporate vice president, head of global therapeutic areas reproductive health and endocrinology at Merck-Serono, a pharmaceutical company, from 2003 until 2007. Prior to this, he was responsible for international marketing and global market development at Biogen Idec, a biotechnology company. Mr Rohde holds a 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, 2013	Supervisory Board	—	296	—	104	—	400
	Management Board	747	377	60	—	—	1,184
	Senior Management	1,101	873	109	—	—	2,083
		1,848	1,546	169	104	—	3,667

(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 30 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. Feczko (Chairman), Ms. Soteropoulos and Mr. Verdonck. Each member satisfies the independence requirements of the NASDAQ listing standards, and Mr. Verdonck qualifies as an "audit committee financial expert," as defined in Item 16A of Form 20-F and as determined by our supervisory board. The audit committee 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), Meyer 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), Meyer 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, 2013, we had a total of 87 employees, of whom 29 had an M.D. or Ph.D. degree, or the foreign equivalent. Of these employees, 20 were engaged in research and development, seven in clinical development, and two in business development functions. We also engaged 33 consultants and contract workers. We do not currently have in place a works council.

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 April 11, 2014 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 17,594,906 ordinary shares outstanding as of April 11, 2014, 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 April 11, 2014 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
Major Shareholders:		
Entities affiliated with Forbion(1)	4,393,523	25.0%
Cooperatieve Gilde Healthcare II U.A.(2)	1,730,415	9.8%
Coller International Partners V-A, L.P.(3)	6,512,043	37.0%
Chiesi Farmaceutici S.p.A.(4)	1,109,214	6.3%
Management Board Members, Supervisory Board Members and Senior Management		
Ferdinand Verdonck(5)	159,826	*
Sander Slootweg(6)	4,393,523	25.0%
Sander van Deventer(7)	4,393,523	25.0%
Joseph M. Feczko(8)	65,275	*
François Meyer(9)	55,279	*
David Schaffer(10)	—	—
Paula Soteropoulos(11)	3,406	—
Jörn Aldag(12)	376,954	2.1%
Piers Morgan(13)	168,444	*
Philip Astley-Sparke(14)	4,505,523	25.6%
Christian Meyer	—	—
Harald Petry(15)	141,279	*
Hans Preusting(16)	143,179	*
Hans Christian Rohde(17)	153,752	*
All current management board members, supervisory board members, and senior management as a group	5,772,917	30.2%
Total shares held by management board members, supervisory board members, senior management and majorshareholders	10,731,066	56.2%

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

(1) Consists of (i) 987,673 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 April 11, 2014 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. Mr. Astley-Sparke, among others, as a venture partner acts as an independent contractor in an advisory function to Forbion Capital Partners. Each of Mr. Slootweg, Dr. van Deventer and Mr. Astley-Sparke disclaim beneficial ownership of such ordinary shares, except to the extent of his pecuniary interest therein. The address of Forbion

Capital Partners, Coöperatieve, FCI, FCI II and SJH is Gooimeer 2-35, 1411 DC Naarden, The Netherlands.

(2) Consists of (i) 1,720,515 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 April 11, 2014 or will become exercisable within 60 days after such date. The manager of Coöperatieve Gilde Healthcare II U.A. is Gilde Healthcare II Management B.V., or Gilde Management, and Gilde Management is owned by Gilde Healthcare Holding B.V., or Gilde Holding. Three managing partners, Edwin de Graaf, Marc Olivier Perret and Martenmanshurk B.V. (of which Pieter van der Meer is the owner and manager) each own 28.66% of Gilde Holding and Stichting Administratiekantoor Gilde Healthcare Holding, or Stichting, owns 14% of Gilde Holding. Stichting is controlled by Mr. de Graaf, Mr. Perret and Martenmanshurk B.V. and issued depository receipts for shares in Gilde Holding to two partners, Arthur Franken and Dirk Kersten. Each of Mr. de Graaf, Mr. Perret and Mr. van der Meer share voting and dispositive power of the shares, and disclaim beneficial ownership of the shares except to the extent of their respective pecuniary interest therein. The address of Coöperatieve Gilde Healthcare II U.A. is Newtonlaan 91, 3584 BP, Utrecht, The Netherlands.

(3) Consists of (i) 2,019,511 ordinary shares held by Coller International Partners V-A, L.P., or Coller; (ii) warrants held by Coller to purchase 99,009 ordinary shares that are exercisable as of April 11, 2014 or will become exercisable within 60 days after such date; (iii) 987,673 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 April 11, 2014 or will become exercisable within 60 days after such date and (vii) 9,859 ordinary shares held by SJH. Coller is a limited partner of the Forbion funds. Coller has no dispositive or voting power over ordinary shares held by the Forbion funds and disclaims beneficial ownership of such ordinary shares except to the extent of its pecuniary interest therein. See footnote 1 above. The general partner of Coller is Coller International General Partner V, L.P. of which Coller Investment Management Limited, or CIML, is the general partner. The directors of CIML are Jeremy Joseph Coller, Cyril Joseph Mahon, Roger Alan Le Tissier, Paul McDonald, Peter Michael Hutton, John Charlton Loveless and Andrew Thane Maden Hitchon and may be deemed to share voting and dispositive power with respect to the ordinary shares held by Coller. The CIML directors disclaim beneficial ownership of such ordinary shares except to the extent of their pecuniary interest therein. The address of Coller is c/o Coller Investment Management Limited, PO Box 255, Trafalgar Court, Les Banques, St Peter Port, Guernsey, Channel Islands.

(4) The registered office of Chiesi Farmaceutici S.p.A is Via Palermo, 26, 43122 Parma, Italy.

(5) Consists of 75,435 ordinary shares and options to purchase 84,391 ordinary shares that are exercisable as of April 11, 2014 or will become exercisable within 60 days after such date.

(6) Consists of securities held by funds affiliated with Forbion. See footnote 1 above.

(7) Consists of securities held by funds affiliated with Forbion. See footnote 1 above.

(8) Consists of 27,768 ordinary shares and options to purchase 37,507 ordinary shares that are exercisable as of April 11, 2014 or will become exercisable within 60 days after such date.

(9) Consists of 17,772 ordinary shares and options to purchase 37,507 ordinary shares that are exercisable as of April 11, 2014 or will become exercisable within 60 days after such date.

(10) Dr. Schaffer joined our supervisory board in January 2014.

(11) Consists of options to purchase 3,406 ordinary shares that are exercisable as of April 11, 2014 or will become exercisable within 60 days after such date.

(12) Consists of 39,389 ordinary shares and options to purchase 337,565 ordinary shares that are exercisable as of April 11, 2014 or will become exercisable within 60 days after such date.

(13) Consists of 27,805 ordinary shares and options to purchase 140,652 ordinary shares that are exercisable as of April 11, 2014 or will become exercisable within 60 days after such date.

(14) Consists of options to purchase 112,000 ordinary shares that are exercisable as of April 11, 2014 or will become exercisable within 60 days after such date, together with securities held by entities affiliated with Forbion. See footnote 1 above.

(15) Consists of 627 ordinary shares and options to purchase 140,652 ordinary shares that are exercisable as of April 11, 2014 or will become exercisable within 60 days after such date.

(16) Consists of 2,527 ordinary shares and options to purchase 140,652 ordinary shares that are exercisable as of April 11, 2014 or will become exercisable within 60 days after such date.

(17) Consists of 13,100 ordinary shares and options to purchase 140,652 ordinary shares that are exercisable as of April 11, 2014 or will become exercisable within 60 days after such date.

Holdings by U.S. Shareholders

As of April 11, 2014, there was one holder of record of ordinary shares (Cede & Co., as nominee for DTC), holding approximately 30.7% of our ordinary shares.

B. Related Party Transactions

Since January 1, 2010, we have engaged in the following transactions with the members of our supervisory board, management board, senior management, holders of 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.

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

2013 Chiesi Collaboration

In June 2013 we completed three inter-conditional agreements with Chiesi; two of these agreements form the basis of our strategic collaboration with Chiesi, as described in "Item 4B—Business Overview". Under the third agreement Chiesi subscribed for 1,109,214 of our ordinary shares at a price of €12.60 per share, raising a total of €14.0 million for uniQure.

For the year ended December 31, 2013 uniQure has received an aggregate amount of €1.2 million from Chiesi under the terms of the strategic collaboration; at December 31, 2013 an amount of €1.4 million was receivable from Chiesi under the strategic collaboration.

2012 and 2013 Convertible Notes

In December 2012, January 2013 and March 2013, we sold convertible promissory notes in the aggregate principal amount of €13.5 million in a private placement to certain of our existing investors, which we refer to as the convertible notes. The convertible notes accrued interest at a rate equal to 8% per year, and had a maturity date of December 31, 2014, unless previously converted. No payments of principal or interest were made under these notes. In addition, in connection with the issuance of the convertible notes we issued the holders of such convertible notes warrants to purchase an aggregate of 133,628 of our ordinary shares. In July 2013, the convertible notes were converted into an aggregate of 1,336,331 of our ordinary shares.

The following table sets forth the participation in this financing by our related parties:

PURCHASER	AGGREGATE PRINCIPAL AMOUNT OF CONVERTIBLE NOTES	ORDINARY SHARES ISSUED UPON CONVERSION OF CONVERTIBLE NOTES	ORDINARY SHARES ISSUABLE UPON EXERCISE OF WARRANTS
Forbion Co-Investment Cooperatief U.A.(1)	€ 1,000,000	99,009	9,900
Cooperatieve Gilde Healthcare II U.A.	€ 1,000,000	99,009	9,900
Coller International Partners V-A, L.P.	€ 10,000,000	990,099	99,009

(1) Sander Slootweg, a member of our supervisory board, is a Managing Partner at Forbion Capital Partners. Sander van Deventer, a member of our supervisory board, is a Managing Partner at Forbion Capital Partners.

2012 Share Purchase Incentive Plan

In November 2012, we raised an aggregate of €552,202 through the issue of ordinary shares at a price of €3.07 per share in part to members of our supervisory board and senior management, including Joseph Feczko, Francois Meyer, Ferdinand Verdonck, Piers Morgan and Hans Christian Rohde. These funds were received in 2012 and 2013.

Grants of Options to Related Parties

The Company 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.

Shareholders Agreements

Our shareholders' agreements related to our class A, B and C shares, were terminated upon the conversion of our class A, B and C shares into ordinary shares on a 1-for-1 basis, effective February 10, 2014.

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 "Business—Strategic Collaboration: 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, we received a formal request for arbitration from Extera Partners, a consulting firm based in Cambridge, Massachusetts, alleging a fee to be due in respect of consulting services provided to us in connection with a partnering transaction. The request for arbitration was received by the International Court of Arbitration at the International Chamber of Commerce on December 12, 2013, which represents the start date of the arbitration. The amount claimed is $100,000 plus 2.5% of all proceeds, including equity investments, we receive from Chiesi pursuant to our collaboration agreements entered into in the second quarter of 2013. Our engagement letter with Extera Partners contains a cap limiting the maximum payment to €5.0 million. We have reviewed this claim with counsel and believe that the claim is without merit. We intend to vigorously defend against it.

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 32 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 4, 2014, the initial trading date for our ordinary shares on NASDAQ through April 11, 2014.

	High	Low
Annual Highs and Lows	$	$
2014 (from February 4, 2014 through April 11, 2014)	18.75	13.00
Annual and Quarterly Highs and Lows		
First Quarter 2014 (from February 4, 2014)	18.75	13.10
Second Quarter 2014 (through April 11, 2014)	16.50	13.00
Monthly Highs and Lows		
February 2014 (from February 4, 2014)	18.75	13.10
March 2014	17.10	14.70
April 2014 (through April 11, 2014)	16.50	13.00

On April 23, 2014, the closing sale price per share on The NASDAQ Global Select Market was $9.62.

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-1 registration statement (File No. 333-193158) originally filed with the SEC on January 2, 2014, as amended. Our articles of association were amended and we converted our company into a public company with limited liability (*naamloze venootschap*) effective February 10, 2014.

C. Material Contracts

We have not entered into any material contracts other than in the ordinary course of business, as described under "Business—Intellectual Property", "—Strategic Collaboration: Chiesi," and "—Strategic Collaboration: 4D Molecular Therapeutics."

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, or 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:

  - If a Shareholder, either alone or, in the case of an individual, together with his partner owns or is deemed to own, directly or indirectly, either a number of shares in us representing five percent or more of our total issued and outstanding capital (or the issued and outstanding capital of any class of our shares), or rights to acquire, directly or indirectly, shares, whether or not already issued, representing five percent or more of our total issued and outstanding capital (or the issued and outstanding capital of any class of our shares), or profit participating certificates (*winstbewijzen*), relating to five percent or more of our annual profit or to five percent of our liquidation proceeds.

  - If the shares, profit participating certificates or rights to acquire shares in us are held or deemed to be held following the application of a non-recognition provision.

  - If the partner of a Shareholder, or one of certain relatives of the Shareholder or of this partner has a substantial interest (as described under 1. and 2. above) in us.

  - A Shareholder receiving income or realizing capital gains in their capacity as future, present or past employee (*werknemer*) or member of a management board (*bestuurder*), or supervisory director (*commissaris*).

  - Pension funds, investment institutions (*fiscale beleggingsinstellingen*), exempt investment institutions (*vrijgestelde beleggingsinstellingen*) and other entities that are exempt from corporate income tax in the Netherlands, as well as entities that are exempt from corporate income tax in their country of residence, such country of residence being another state of the European Union, Norway, Liechtenstein, Iceland or any other state with which the Netherlands have agreed to exchange information in line with international standards.

For purposes of Dutch personal income tax and Dutch corporate income tax, ordinary shares legally owned by a third party, such as a trustee, foundation or similar entity or arrangement, may under certain circumstances have to be allocated to the (deemed) settler, grantor or similar organiser

or, upon the death of the Settlor, his/her beneficiaries in proportion to their entitlement to the estate of the Settlor of such trust or similar arrangement.

This summary is based on the tax laws and principles (unpublished case law not included) in the Netherlands as in effect on the date of this 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, whatever they may be named or in whatever form;
- liquidation proceeds, or proceeds from the repurchase of ordinary shares by us in excess of the average paid-in capital recognized for Dutch dividend withholding tax purposes;
- the par value of ordinary shares issued to a Shareholder in us or an increase of the par value of ordinary shares, to the extent that it does not appear that a contribution, recognized for Dutch dividend withholding tax purposes, has been made or will be made; and
- partial repayment of share capital, if and to the extent that there are net profits (zuivere winst), unless (a) the general meeting of shareholders has resolved in advance to make such repayment and (b) the par value of the shares concerned has been reduced by an equal amount by way of an amendment to our articles of association.

In general, we will be required to remit all amounts withheld as Dutch dividend withholding tax to the Dutch tax authorities. However, under certain circumstances, we are allowed to reduce the amount to be remitted to the Dutch tax authorities by the lesser of:

- 3% of the portion of the distribution paid by us that is subject to Dutch dividend withholding tax; and
- 3% of the dividends and profit distributions, before deduction of foreign withholding taxes, received by us from qualifying foreign subsidiaries in the current calendar year (up to the date of the distribution by us) and the two preceding calendar years, as far as such dividends and profit distributions have not yet been taken into account for purposes of establishing the above mentioned reduction.

Although this reduction reduces the amount of Dutch dividend withholding tax that we are required to remit to the Dutch tax authorities, it does not reduce the amount of tax that we are required to withhold on dividends distributed.

Residents of the Netherlands

A Shareholder which is resident or deemed resident in the Netherlands is generally entitled to a full credit of any Dutch dividend withholding tax against the Dutch (corporate) income tax liability of such Shareholder, and is generally entitled to a refund in the form of a negative assessment of Dutch (corporate) income tax, insofar such Dutch dividend withholding tax, together with any other creditable domestic and/or foreign taxes, exceeds such Shareholder's aggregate Dutch income tax or Dutch corporate income tax liability.

If and to the extent that such a corporate Shareholder is eligible for the application of the participation exemption with respect to the ordinary shares, dividends distributed by us are in principle exempt from Dutch dividend withholding tax.

Pursuant to domestic anti-dividend stripping rules, no exemption from Dutch dividend withholding tax, credit against Dutch (corporate) income tax, refund or reduction of Dutch dividend withholding tax shall apply if the recipient of the dividend paid by us is not considered to be the beneficial owner (*uiteindelijk gerechtigde*) as meant in these rules, of such dividends.

Non-residents of the Netherlands (including but not limited to U.S. Shareholders)

A non-resident Shareholder, which is resident in the non-European part of the Kingdom of the Netherlands or in a country that has concluded a tax treaty with the Netherlands, may be eligible for a full or partial relief from Dutch dividend withholding tax, provided such relief is timely and duly claimed.

In addition, a non-resident Shareholder that is not an individual, is entitled to an exemption from Dutch dividend withholding tax, provided that each of the following tests are satisfied:

- the non-resident Shareholder is, according to the tax law of a Member State of the European Union or a state designated by a ministerial decree, that is a party to the Agreement regarding the European Economic Area, resident there and it is not transparent for tax purposes according to the tax law of such state;

- anyone or more of the following threshold conditions are satisfied:

    - at the time the dividend is distributed by us, the non-resident Shareholder holds shares representing at least five percent of our nominal paid-up capital; or

    - the non-resident Shareholder has held shares representing at least five percent of our nominal paid-up capital for a continuous period of more than one year at any time during four years preceding the time the dividend is distributed by us; or

    - the non-resident Shareholder is connected with us within the meaning of article 10a, paragraph 4 of the Dutch Corporate Income Tax Act 1969 (Wet op de vennootschapsbelasting 1969, or CITA); or

    - an entity connected with the non-resident Shareholder within the meaning of article 10a, paragraph 4 of CITA holds at the time of the dividends distributed by us, shares representing at least five per cent of our nominal paid-up capital; and

- the non-resident Shareholder is not considered to be resident outside the Member States of the European Union or the states designated by ministerial decree, that are party to the Agreement regarding the European Economic Area, under the terms of a tax treaty concluded with a third state.

A non-resident Shareholder which is resident in a Member State of the European Union with which the Netherlands has concluded a tax treaty that provides for a reduction of Dutch tax on dividends based on the ownership of the number of voting rights, the test under 2.a. above is also satisfied if the non-resident Shareholder owns at least five percent of the voting rights in us.

The exemption from Dutch dividend withholding tax is not available to a non-resident Shareholder if pursuant to a provision for the prevention of fraud or abuse included in a tax treaty between the Netherlands and the country of residence of the non-resident Shareholder, the non-resident Shareholder is not entitled to the reduction of Dutch tax on dividends provided for by such treaty.

Furthermore, pursuant to domestic anti-dividend stripping rules, no exemption from Dutch dividend withholding tax, refund or reduction of Dutch dividend withholding tax shall apply if the recipient of the dividend paid by us is not considered to be the beneficial owner (*uiteindelijk gerechtigde*) as meant in these rules, of such dividends. The Dutch tax authorities have taken the position that this beneficial ownership test can also be applied to deny relief from Dutch dividend withholding tax under tax treaties and the Tax Arrangement for the Kingdom (*Belastingregeling voor het Koninkrijk*).

A non-resident Shareholder which is subject to Dutch income tax or Dutch corporate income tax in respect of any benefits derived or deemed to be derived from ordinary shares, including any capital gain realized on the disposal thereof, can generally credit Dutch dividend withholding tax against its Dutch income tax or its Dutch corporate income tax liability, as applicable, and is generally entitled to a refund pursuant to a negative tax assessment if and to the extent the Dutch dividend withholding tax, together with any other creditable domestic and/or foreign taxes, exceeds its aggregate Dutch income tax or its aggregate Dutch corporate income tax liability, respectively.

Taxes on Income and Capital Gains

Residents of the Netherlands

Individuals

A Shareholder, who is an individual resident or deemed to be resident in the Netherlands, or who has elected to be taxed as a resident of the Netherlands for Dutch personal income tax purposes, will be subject to regular Dutch personal income tax at progressive rates (up to a maximum rate of 52%) under the Dutch Income Tax Act 2001 on the income derived from the ordinary shares and gains realized on the disposal thereof if:

- such Shareholder derives any benefits from the ordinary shares, which are attributable to an enterprise of such Shareholder, whether as an entrepreneur or pursuant to a co-entitlement to the net worth of an enterprise, other than as a shareholder or an entrepreneur; or

- such income or gain is taxable in the hands of such Shareholder as benefits from miscellaneous activities (*resultaat uit overige werkzaamheden*), including but not limited to activities with respect to the ordinary shares that are beyond the scope of regular active portfolio management activities.

If neither of the two abovementioned conditions apply, such Shareholder must determine his or her taxable income with regard to the ordinary shares on the basis of a deemed return on income from savings and investments (*sparen en beleggen*), rather than on the basis of income actually received or gains actually realized. This deemed return on income from savings and investments has been fixed at a rate of 4% of the individual's yield basis at the beginning of the calendar year, insofar as the individual's yield basis exceeds a certain threshold. The individual's yield basis is determined as the fair market value of certain qualifying assets held by the individual less the fair market value of certain qualifying liabilities at the beginning of the calendar year.

Corporate entities

Generally, corporate Shareholders that are resident or deemed to be resident in the Netherlands for Dutch corporate income tax purposes will be subject to regular Dutch corporate income tax, levied at a rate of 25% (20% over profits up to €200,000) over income derived from the ordinary shares and gains realized upon acquisition, redemption and disposal of ordinary shares.

If and to the extent that such Shareholder is eligible for the application of the participation exemption with respect to the ordinary shares, income derived from the ordinary shares and gains and

losses (with the exception of liquidation losses under strict conditions) realized on the ordinary shares may be exempt from Dutch corporate income tax.

Non-residents of the Netherlands (including but not limited to U.S. Shareholders)

Individuals

A Shareholder, who is an individual not resident or deemed to be resident in the Netherlands, and who has not elected to be taxed as a resident of the Netherlands for Dutch income tax purposes, will not be subject to any Dutch taxes on income or capital gains in respect of dividends distributed by us or in respect of any gain realized on the disposal of ordinary shares (other than dividend withholding tax as described above), except if:

- such holder has an enterprise or an interest in an enterprise that is, in whole or in part, carried on through a permanent establishment or a permanent representative in the Netherlands and to which enterprise or part of an enterprise, as the case may be, the ordinary shares are attributable; or

- such income or gain such income or gain is taxable in the hands of such Shareholder as benefits from miscellaneous activities" (resultaat uit overige werkzaamheden), including but not limited to activities with respect to the ordinary shares that are beyond the scope of regular active portfolio management.

If one of the two abovementioned conditions apply, the income or gains in respect of dividends distributed by us or in respect of any capital gain realized on the disposal of ordinary shares will in general be subject to Dutch personal income tax at the progressive rates up to 52%.

Corporate entities

A corporate Shareholder, which is not resident or deemed to be resident in the Netherlands for Dutch corporate income tax purposes, will not be subject to any Dutch taxes on income or capital gains in respect of dividends distributed by us, or in respect of any gain realized, on the disposal of ordinary shares (other than dividend withholding tax as described above), except if:

- such Shareholder has an enterprise or an interest in an enterprise that is, in whole or in part, carried on through a permanent establishment or a permanent representative in the Netherlands, to which the ordinary shares are attributable; or

- such holder has a substantial interest or a deemed substantial interest in us (as described above), that (i) is held with the evasion of income tax or dividend withholding tax as (one of) the main purpose(s) and (ii) is not attributable to the assets of an enterprise of such Shareholder; or

- such holder is an entity resident of Aruba, Curaçao or Saint Martin with a permanent establishment or permanent representative in Bonaire, Saint Eustatius or Saba to which such income or gain is attributable, and the permanent establishment or permanent representative would be deemed to be resident of the Netherlands for Dutch corporate income tax purposes (i) had the permanent establishment been a corporate entity (lichaam), or (ii) had the activities of the permanent representative been conducted by a corporate entity, respectively.

If one of the abovementioned conditions applies, income derived from the ordinary shares and gains realized on ordinary shares will, in general, be subject to regular Dutch corporate income tax levied at a rate of 25% (20% over profits up to €200,000), except that a holder referred to under (2) above will generally be subject to an effective corporate income tax rate of 15% if it holds the substantial interest in us only with the purpose of avoiding dividend withholding tax and not with (one of) the main purposes to avoid income tax.

Gift or Inheritance Taxes

No Dutch gift or Dutch inheritance tax is due in respect of any gift, in form or in substance, of the ordinary shares by, or inheritance of the shares on the death of, a Shareholder except if:

- at the time of the gift or death of the Shareholder, the Shareholder is resident, or deemed to be resident, in the Netherlands for purposes of Dutch gift tax or Dutch inheritance tax, as applicable; or

- in the case of a gift of ordinary shares by an individual who at the date of the gift was neither resident nor deemed to be resident in the Netherlands (i) such individual dies within 180 days after the date of the gift, while being resident or deemed to be resident in the Netherlands; or (ii) the gift of ordinary shares is made under a condition precedent and the Shareholder is resident, or is deemed to be resident in the Netherlands at the time the condition is fulfilled.

For purposes of the above, a gift of ordinary shares made under a condition precedent (*opschortende voorwaarde*) is deemed to be made at the time the condition precedent is satisfied.

For purposes of Dutch gift or Dutch inheritance taxes, an individual not holding the Dutch nationality will be deemed to be resident in the Netherlands, *inter alia*, if he or she has been resident in the Netherlands at any time during the ten years preceding the date of the gift or his or her death. Additionally, for purposes of Dutch gift tax, an individual not holding the Dutch nationality will be deemed to be resident in the Netherlands if he or she has been resident in the Netherlands at any time during the twelve months preceding the date of the gift. Applicable tax treaties may override deemed residency in the Netherlands.

Value Added Tax

No Dutch value added tax will arise in respect of payments in consideration for the issue, acquisition, ownership and disposal of ordinary shares, other than value added taxes on fees payable in respect of services not exempt from Dutch value added tax.

Other Taxes and Duties

No Dutch registration tax, capital tax, custom duty, transfer tax, stamp duty or any other similar tax or duty, other than court fees, will be payable in the Netherlands in respect of or in connection with the subscription, issue, placement, allotment, delivery or transfer of the ordinary shares.

Residence

A Shareholder will not become resident, or deemed resident in the Netherlands for tax purposes by reason only of holding the ordinary shares.

Taxation in the United States

The following summary of the material U.S. federal income tax consequences of the acquisition, ownership and disposition of our ordinary shares is based upon current law and does not purport to be a comprehensive discussion of all the tax considerations that may be relevant to 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 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.

An additional 3.8% tax is imposed on the net investment income (which includes taxable dividends and net capital gains) received by certain U.S. holders that are individuals, trusts or estates.*Passive foreign investment company considerations.* A corporation organized outside the United States generally will be classified as a passive foreign investment company, or PFIC, for U.S. federal income tax purposes in any taxable year in which either: (i) at least 75% of its gross income is passive income, or (ii) on average at least 50% of the gross value of its assets is attributable to assets that produce passive income or are held for the production of passive income. In arriving at this calculation, a pro rata portion of the income and assets of each corporation in which we own, directly or indirectly, at least a 25% interest, as determined by the value of such corporation, must be taken into account. Passive income for this purpose generally includes dividends, interest, royalties, rents and gains from commodities and securities transactions.

We believe that we were not a PFIC for the 2013 taxable year. Based on our estimated gross income, the average value of our gross assets, and the nature of the active businesses conducted by our "25% or greater" owned subsidiaries, we do not believe that we will be classified as a PFIC in the current taxable year and do not expect to become one in the foreseeable future. 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 the 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 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. To date, we have used €9.3 million of the net proceeds of the offering, including €4.3 million to complete the building out and equipping of our manufacturing facility in Lexington. We intend to use the remaining net proceeds to complete the building out and equipping of our manufacturing facility in Lexington, Massachusetts; to support our further clinical development of Glybera, and our application for marketing approval of Glybera and preparation for potential commercial launch in the United States; to fund our share of the costs of our planned Phase I/II clinical trial of AMT 060 in hemophilia B; to advance the development of our other product candidates and research activities, including our collaboration with 4D Molecular Therapeutics; and for working capital and for general corporate purposes, including the costs of operating our

facilities in Amsterdam and in Lexington, Massachusetts, service on our indebtedness and potentially acquisitions or investments in other businesses, technologies or product candidates. Our management retains broad discretion in the allocation and use of the remaining net proceeds of our initial public offering. Pending such decisions, we have invested such proceeds in investment grade, interest bearing securities or bank deposits.

Item 15 ***Control and Procedures***

The company's management, with the participation of the company's chief executive officer and chief financial officer, evaluated the effectiveness of the company's disclosure controls and procedures as of December 31, 2013. 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. Disclosure controls and procedures include, without limitation, controls and procedures 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 accumulated and communicated to the company's management, including its principal executive and principal financial officers, as appropriate to allow timely decisions regarding required disclosure. Based on the evaluation of the company's disclosure controls and procedures as of December 31, 2013, the company's chief executive officer and chief financial officer concluded that, as of such date, the company's disclosure controls and procedures were not effective as a result of the material weaknesses in internal control described below.

This annual report does not include a report of management's assessment regarding internal control over financial reporting or an attestation report of the company's independent registered public accounting firm due to a transition period established by rules of the Securities and Exchange Commission for newly public companies.

Neither we nor our independent registered public accounting firm undertook a comprehensive assessment of our internal control for purposes of identifying and reporting material weaknesses, significant deficiencies and control deficiencies in our internal control over financial reporting. In its review of internal control over financial reporting in connection with the company's initial public offering in February 2014, management identified three material weaknesses:

- a lack of accounting resources required to fulfill IFRS and SEC reporting requirements;
- a lack of comprehensive IFRS accounting policies and financial reporting procedures; and
- a lack of segregation of duties given the size of our finance and accounting team.

We have implemented and are continuing to implement various measures to address the material weaknesses identified; these measures are outlined in "Operating and Financial Review and Prospects—Internal Control Over Financial Reporting".

We believe it is possible that, had we performed a formal assessment of our internal control over financial reporting or had our independent registered public accounting firm performed an audit of our internal control over financial reporting, additional control deficiencies may have been identified.

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, other than as described in "Operating and Financial Review and Prospects-Internal Control Over Financial Reporting."

Item 16A ***Audit Committee Financial Expert***

Mr. Ferdinand Verdonck, an independent director and a member of the Audit Committee, qualifies 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

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, 2011		Year ended December 31, 2012		Year ended December 31, 2013	
	EUR'000	%	EUR'000	%	EUR'000	%
Audit Fees	167	66%	65	93%	1,021	98%
Audit-related Fees	46	18%	—	—%	—	0%
Tax Fees	39	16%	5	7%	20	2%
Total	252	100%	70	100%	1,041	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. The policy was not in place during 2013.

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 "Item 18 Financial Statements."

Item 18 ***Financial Statements***

See the Financial Statements beginning on page F-1.

Item 19 ***Exhibits***

Exhibit No.	Description
1.1*	Amended Articles of Association of the Company
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 Asklêpios 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).
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).

Exhibit No.	Description
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 Applicada, 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).
4.17	Loan and Security Agreement, dated as of June 13, 2013, by and among the Company, uniQure IP B.V., the Company's subsidiaries listed therein, and Hercules Technology Growth Capital, Inc. (incorporated by reference to Exhibit 10.17 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).
8.1*	Subsidiaries of the Company (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).
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

Exhibit No.	Description
13.1*	Certification by Principal Executive Officer and Principal Financial Officer Pursuant to Section 906 of the Sarbanes-Oxley Act of 2002

† Confidential treatment requested as to certain portions, which portions have been omitted and filed separately with the Securities and Exchange Commission

* Filed herewith

Table of Contents

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

Mr Jörn Aldag
Managing Director/Chief Executive Officer

Date: April 25, 2014

By: /s/ PIERS MORGAN

Mr Piers Morgan
Managing Director/Chief Financial Officer

Date: April 25, 2014

UNIQURE N.V.

INDEX TO CONSOLIDATED FINANCIAL STATEMENTS

REPORT OF INDEPENDENT REGISTERED PUBLIC ACCOUNTING FIRM

To 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, 2013 and December 31, 2012, and the results of their operations and their cash flows for each of the three years in the period ended December 31, 2013 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 25, 2014

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

UNIQURE N.V.

Consolidated Balance Sheets

(€ in thousands)

	NOTE	As of December 31, 2012	2013
Assets			
Non-current assets			
Intangible assets	*5*	3,278	7,775
Property, plant and equipment	*6*	1,185	2,614
Other non-current assets	*7*	—	923
Total non-current assets		4,463	11,312
Current assets			
Receivables from related parties	*8,29*	26	1,425
Trade and other receivables	*8*	815	1,557
Inventories	*9*	—	865
Cash and cash equivalents	*10*	263	23,810
Total current assets		1,104	27,657
Total assets		5,567	38,969
(Deficit)/Equity			
Share capital		483	610
Share premium		114,795	142,459
Other reserves		1,508	6,536
Accumulated deficit		(117,234)	(144,041)
Total (Deficit)/equity	*11*	(448)	5,564
Liabilities			
Non-current liabilities			
Borrowings	*16*	—	6,292
Financial lease liabilities	*13*	450	302
Deferred rent	*8,28*	—	680
Deferred revenue	*17*	—	15,679
Total non-current liabilities		450	22,953
Current liabilities			
Trade and other payables	*15*	4,067	7,601
Debt to related party—financial liability	*14*	1,366	—
Debt to related party—embedded derivative	*14*	132	722
Borrowings	*16*	—	633
Borrowings—embedded derivative	*16*	—	217
Deferred revenue	*17*	—	1,279
Total current liabilities		5,565	10,452
Total liabilities		6,015	33,405
Total equity and liabilities		5,567	38,969

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

UNIQURE N.V.

Consolidated Statements of Comprehensive Loss

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

	NOTE	YEARS ENDED DECEMBER 31, 2011	2012	2013
License revenues	*17*	—	—	440
Collaboration revenues	*17*	—	—	2,503
Total revenues		—	—	2,943
Cost of goods sold	*28*	—	—	(800)
Other income	*18*	2,192	649	585
Research and development expenses	*19,20,23*	(15,500)	(10,231)	(13,182)
Selling, general and administrative expenses	*19,21,23*	(3,807)	(4,564)	(11,628)
Other losses, net	*18*	(26)	(45)	(453)
Total Operating Costs		(19,333)	(14,840)	(25,263)
Operating result		(17,141)	(14,191)	(22,535)
Finance income	*24*	277	22	102
Finance expense	*24*	(436)	(547)	(4,387)
Finance income/(expense)—net		(159)	(525)	(4,285)
Result before corporate income taxes		(17,300)	(14,716)	(26,820)
Corporate income taxes		—	—	—
Net Loss		(17,300)	(14,716)	(26,820)
Items that may be subsequently reclassified to profit or loss	*22*	—	—	12
Other comprehensive income		—	—	—
Total comprehensive loss*		(17,300)	(14,716)	(26,808)
Loss per share attributable to the equity holders of the Company during the year				
Basic and diluted loss per share (in euro)	*26*	(3.65)	(1.70)	(2.48)

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

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

UNIQURE N.V.

Consolidated Statements of Changes in (Deficit)/Equity

(€ in thousands)

	NOTE	ATTRIBUTABLE TO EQUITY HOLDERS OF THE COMPANY				
		SHARE CAPITAL	SHARE PREMIUM RESERVE	OTHER RESERVES	ACCUMULATED DEFICIT	TOTAL (DEFICIT)/ EQUITY
Balance at January 1, 2011		235	99,841	1,788	(88,205)	13,659
Result for the year		—	—	—	(17,300)	(17,300)
Capital contributions	*11*	2	106	—	—	108
Share-based payment expenses	*11*	—	—	940	—	940
Balance at December 31, 2011		237	99,947	2,728	(105,505)	(2,593)
Result for the year					(14,716)	(14,716)
Capital contributions	*11*	246	14,848	—	—	15,094
Share-based payment expenses relating to the AMT share option scheme	*12*	—	—	259	—	259
Adjustment to reserves on expiration of the AMT option scheme	*11*	—	—	(2,987)	2,987	—
Share-based payment expenses relating to the uniQure share option scheme	*12*	—	—	1,508	—	1,508
Balance at December 31, 2012		483	114,795	1,508	(117,234)	(448)
Result for the year		—	—	—	(26,820)	(26,820)
Other Comprehensive Income	*22*	—	—	—	12	12
Capital contributions	*11*	127	27,664	—	—	27,791
Result on conversion of loan	*14*	—	—	3,005	—	3,005
Share-based payment expenses	*12*	—	—	2,023	—	2,023
Balance at December 31, 2013		610	142,459	6,536	(144,041)	5,564

The notes are an integral part of these consolidated financial statements

UNIQURE N.V.

Consolidated Statement of Cash Flows

(€ in thousands)

	NOTE	YEARS ENDED DECEMBER 31, 2011	2012	2013
Cash flow from operating activities				
Result before corporate income tax		(17,300)	(14,716)	(26,820)
Adjustments for:				
—Depreciation	*6*	590	548	535
—Impairment of assets	*5*	300	—	—
—Lease Incentive	*28*	—	—	134
—Derivative result	*14*	(207)	(22)	2,113
—Derivative result arising on early conversion of a loan	*14*	—	464	1,333
—Exchange result		26	45	49
—Share-based payment expenses	*12*	940	1,767	2,023
—Changes in other non-current assets	*7*	—	—	(923)
—Changes in trade and other receivables		(442)	243	(1,439)
—Movement in inventories	*9*	—	—	(865)
—Changes in trade and other payables		(1,039)	180	359
—Changes in deferred revenue and provisions	*17*	—	—	16,958
—Movement in other liabilities		64	161	2,052
—Interest (income)/expense		365	61	1,244
Cash used in operations		(16,703)	(11,269)	(3,247)
Interest paid		(2)	(8)	(889)
Net cash used in operating activities		(16,705)	(11,277)	(4,136)
Cash flow from investing activities				
Purchases of property, plant and equipment	*6*	(200)	(392)	(1,336)
Purchases of intangible assets	*5*	(109)	(553)	(4,652)
Interest received		147	113	17
Net cash used in investing activities		(162)	(832)	(5,971)
Cash flow from financing activities				
Capital contribution from shareholders	*11*	108	9,774	14,294
Convertible loans drawn down	*11,14*	—	1,498	11,999
Proceeds from borrowings	*16*	—	—	7,492
Redemption of financial lease	*13*	—	—	(143)
Net cash generated from financing activities		108	11,272	33,642
Net increase in cash, cash equivalents, and other bank overdrafts		(16,759)	(837)	23,535
Currency effect cash and cash equivalents	*22*	—	—	12
Cash, cash equivalents, and other bank overdrafts at beginning of the period		17,859	1,100	263
Cash, cash equivalents, and other bank overdrafts cash at end of the period	*10*	1,100	263	23,810

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

UNIQURE N.V.
NOTES TO CONSOLIDATED FINANCIAL STATEMENTS
FOR THE YEARS ENDED DECEMBER 31, 2011, 2012 AND 2013

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 DR holders; the STAK Trustees attend uniQure shareholder meetings on behalf of the uniQure DR holders and will follow voting instructions from the uniQure DR holders in respect of any resolutions at shareholder meetings.

These consolidated financial statements of the Company are prepared on a going concern basis taking into account the successful completion of its initial public offering on February 5, 2014 generating net proceeds of €62.6 million after commissions but before expenses.

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.

Significant shareholders

The Company's significant shareholders* as at December 31, 2013 were:

Advent Venture Partners
Coller Capital
Chiesi Farmaceutici S.p.A
Forbion Capital Partners
Gilde Healthcare Partners
Grupo Netco and affiliates
Lupus Alpha PE Champions
Omnes Capital (formerly Credit Agricole Private Equity)

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* Following the IPO which took place on February 5, 2014, uniQure no longer regards these investors as significant shareholders holding more than 5% of the Company's shares.

Organizational structure of the uniQure Group

uniQure N.V. is the ultimate parent of the following entities which were transferred to uniQure's ownership as part of the transaction with AMT (as described above) and which were renamed following the transaction, as follows:

Company name	Formerly known as
uniQure biopharma B.V.	Amsterdam Molecular Therapeutics (AMT) B.V.
uniQure IP B.V.	Amsterdam Molecular Therapeutics (AMT) IP B.V.
uniQure Manufacturing B.V.	AMT manufacturing B.V.
uniQure Assay Development B.V.	AMT Assay Development B.V.
uniQure Research B.V.	AMT Research B.V.
uniQure non clinical B.V.	AMT non clinical B.V.
uniQure QA B.V.	AMT QA B.V.
uniQure Process Development B.V.	AMT Process Development B.V.
uniQure clinical B.V.	AMT clinical B.V.
Stichting participatie AMT(1)	Stichting participatie AMT(1)
uniQure Inc.(2)	

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(1) Stichting participatie AMT is a Trust, not a company, but met the conditions for consolidation within uniQure's consolidated financial statements. Stichting participatie AMT was established to facilitate AMT's employee incentive schemes for the period up to 2010.

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

Other matters

The Company's business is not subject seasonal influences.

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, for 2011, 2012 and 2013, presented in these consolidated financial statements and accompanying footnotes has been retroactively adjusted, where applicable, to reflect the impact of the reverse share split.

At the time of the initial public offering all existing shareholders agreed to a 180 day lock-up that will expire on August 4, 2014.

The consolidated financial statements were authorized for issue by the supervisory board on April 14, 2014.

2. Summary of Significant Accounting Policies

Introductory notes on the basis of preparation and presentation of the financial statements

As described in Note 1 above, the combination of uniQure and the AMT Business in 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 12 months ended December 31, 2012 (including the results of AMT prior to its acquisition by uniQure) and for the 12 months ended December 31, 2013.

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.

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.

Under IFRS 3, the acquisition of the AMT Business by uniQure from AMT, which was completed on April 5, 2012, is accounted for as a reverse acquisition; therefore, the financial information is presented on a continuing basis for the AMT Business and uniQure. Under IFRS 3 uniQure is the legal parent of the AMT Business but is regarded as the accounting acquiree; conversely the AMT Group is the legal subsidiary but the accounting acquirer in the consolidated financial statements.

2.2 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, 2013 and have been adopted by the Company in the preparation of the consolidated financial statements:

- Amendment to IFRS 7 Financial instruments—disclosures
- IFRS 10 Consolidated financial statements
- IFRS 11 Joint arrangements
- IFRS 12 Disclosures of interest in other entities
- IFRS 13 Fair value measurement
- Amendment to IAS 1 Presentation of financial statements
- Improvements to IAS 16 Property plant and equipment
- Amendment to IAS 19 Employee benefits
- IAS 27 (revised 2011) Separate financial statements
- IAS 28 (revised 2011) Investments in associates and joint ventures
- Improvements to IAS 32 Financial statements—presentation
- Improvements to IAS 34 Interim financial reporting
- IFRIC 21 Levies[(1)]

The adoption of these new standards and amendments did not materially impact the Company's financial position or results of operations.

(b) New and amended standards not yet adopted by the Company

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 are expected to have a material effect on the consolidated financial statements of the Company.

The standards which could have a significant effect on the consolidated financial statements of the Company are IFRS 9 "Financial Instruments" and Amendments to IAS 36 "Impairment of Assets". IFRS 9 is the first step in the process of replacing IAS 39 "Financial Instruments: Recognition and Measurement". The Company has yet to assess IFRS 9's full impact. Amendments to IAS 36 removes certain disclosures of the recoverable amount of Cash Generating Units which had been included in IAS 36 by the issue of IFRS 13. The Company has yet to assess Amendments to IAS 36's full impact.

The IASB has also issued Exposure Drafts in which significant changes on accounting and disclosures are proposed on topics such as lease accounting and revenue recognition. If the current proposals lead to new or amended standards, the changes could have a substantial impact on uniQure's financial statements in the coming years. The effective date of the revised standards is still under discussion.

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(1) Applicable for accounting periods beginning on or after January 1, 2014, however uniQure has adopted this standard early.

2.3 Consolidation

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.

Inter-company transactions, balances, income and expenses on transactions between uniQure companies are eliminated. Profits and losses resulting from inter-company transactions that are recognized in assets are also eliminated. Accounting policies of subsidiaries have been changed where necessary to ensure consistency with the policies adopted by the Company.

2.4 Segment Reporting

Operating segments are identified on the basis of whether the allocation of resources and/or the assessment of performance of a particular component of uniQure's activities are regularly reviewed by uniQure's chief operating decision maker as a separate operating segment. By these criteria, the activities of uniQure are considered to be one segment, which comprises the discovery, development and commercialization of innovative gene therapies and the segmental analysis is the same as the analysis for uniQure as a whole. The Management Board is identified as the chief operating decision maker, and reviews the consolidated operating results regularly to make decisions about the resources and to assess overall performance.

The Company currently, and in the near future, is expected to derive the substantial majority of its revenues from a single party, Chiesi, based in Italy. The Company and Chiesi have entered into an exclusive collaboration for the development and commercialization of the Company's Glybera and Hemophilia B programs in Europe and certain additional territories, pursuant to agreements which were entered into in April 2013, and which became effective in June 2013.

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.

2.6 Notes to the cash flow statement

The cash flow statement has been prepared using the indirect method. The cash disclosed in the cash flow statement is comprised of cash and cash equivalents. Cash comprises cash on hand and demand deposits. Cash equivalents are short- term, highly liquid investments that are readily convertible to known amounts of cash and are subject to an insignificant risk of changes in value. Cash

flows denominated in foreign currencies have been translated at the average exchange rates. Exchange differences, if any, affecting cash items are shown separately in the cash flow statement. Interest paid and received, dividends received and income tax are included in the cash from operating activities.

Further details are set out in Note 10 below.

2.7 Intangible Assets

(a) Licenses

Acquired patents have a definite useful life and are carried at cost less accumulated amortization and impairment losses. Amortization is calculated using the straight-line method to allocate the cost of licenses over their estimated useful lives (generally 20 years unless a license expires prior to that date). Amortization begins when an asset is available for use.

(b) Research and Development

Research expenditures are recognized as expenses as incurred. Costs incurred on development projects are recognized as intangible assets as of the date that it can be established that it is probable that future economic benefits that are attributable to the asset will flow to the Company considering its commercial and technological feasibility, generally when a filing is made for regulatory approval for commercial production, and when costs can be measured reliably.

2.8 Property, Plant and Equipment

Property, plant and equipment comprise mainly laboratory equipment, leasehold improvements, furniture and computer hardware/software. All property, plant and equipment are stated at historical cost less depreciation. Historical cost includes expenditures that are directly attributable to the acquisition of the items.

Subsequent costs are included in the asset's carrying amount or recognized as a separate asset, as appropriate, only when it is probable that future economic benefits associated with the item will flow to uniQure and the cost of the item can be measured reliably. All other repairs and maintenance charges are expensed in the period in which such charges are incurred.

Depreciation is calculated using the straight-line method to allocate the cost of the assets to their residual values over their estimated useful lives. Property, plant and equipment are depreciated as follows:

- Leasehold improvements periods between 5 - 15 years
- Laboratory equipment periods between 5 - 10 years
- Computer hardware/software 3 years

The assets' residual values and useful lives are reviewed, and adjusted if appropriate, at each balance sheet date. An asset's carrying amount is written down immediately to its recoverable amount if the asset's carrying amount is greater than its estimated recoverable amount.

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 2.23 below.

2.9 Impairment of Non-Financial Assets

Assets that are not subject to amortization (whether or not they are ready for use) are tested annually for impairment. Assets that are subject to amortization are reviewed for impairment whenever events or changes in circumstances indicate that the carrying amount may not be recoverable. 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 fair value of a ordinary share) 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 that have been previously impaired are reviewed for possible reversal of the impairment at each subsequent reporting date.

2.10 Recognition and measurement

Financial assets and financial liabilities are included in uniQure's balance sheet when uniQure becomes a party to the contractual provisions of the instrument. Financial assets are derecognized when the rights to receive cash flows from the investments have expired or have been transferred and the company has transferred substantially all risks and rewards of ownership.

Non-derivative financial instruments

Cash and cash equivalents

Cash and cash equivalents includes bank balances, demand deposits and other short-term, highly liquid investments (with less than three months to maturity) that are readily convertible into a known amount of cash and are subject to an insignificant risk of fluctuations in value.

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.

2.11 Offsetting financial instruments

Financial assets and liabilities are offset and the net amount is reported in the balance sheet when there is a legally enforceable right to offset the recognized amounts and there is an intention to settle on a net basis, or realize the asset and settle the liability simultaneously.

2.12 Inventories

Inventories are stated at the lower of cost and net realizable value. Cost is determined using the first-in, first-out (FIFO) method. The cost of finished goods and work in progress comprises design costs, raw materials, direct labor, other direct costs and related production overheads (based on normal operating capacity). It excludes borrowing costs. Net realizable value is the estimated selling price in the ordinary course of business, less applicable variable selling expenses.

2.13 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.

An equity instrument is defined as any contract that evidences a residual interest in the assets of an entity after deducting all of its liabilities. An instrument is an equity instrument only if the issuer has an unconditional right to avoid settlement in cash or another financial asset.

Ordinary Shares

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

Convertible Loan

Where the Company issues convertible loans that do not have the unconditional right to avoid delivering cash or a variable number of shares to settle obligations towards loan note holders, the Company accounts for such loan notes as containing an element that qualifies as a financial liability. Convertible loans are split into a debt component and a separate conversion option component. The conversion option is recognized initially at fair value, based on a probability-weighted scenario analysis. The debt component is the residual amount after deducting from the fair value of the loan as a whole (i.e. the issuance proceeds) the amount separately determined for the conversion option component. The debt component is subsequently carried at amortized cost using the effective interest rate method. When estimates regarding the amount or timing of payments required to settle the obligation change, the carrying amount of the financial liability is adjusted to reflect actual and revised estimated cash flows. The carrying amount is recalculated by computing the present value of estimated future cash flows at the financial instrument's original effective interest rate. Such adjustments are recognized as income or expense in the income statement. Any incremental costs of the loan are deducted from the carrying amount and are amortized over the term of the convertible loan under the effective interest rate method.

The conversion option is classified as a liability if it may be settled by either party other than by the exchange of a fixed amount of cash for a fixed number of the entity's own equity instruments. In that case, the conversion option is carried at fair value with changes in fair value recorded in the income statement. If the conversion option qualifies as an equity instrument, it is recognized in equity on issue date and not re-measured.

2.14 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.

2.15 Deferred Corporate Income Taxes

There is no tax charge in the Company's Consolidated Statements of Comprehensive Income, nor any deferred tax recognized in the balance sheet for the periods covered by these financial statements.

To the extent that any tax expense would arise, it would comprise current and deferred tax. Tax effects are recognized in the income statement, except to the extent that they relate to items recognized in other comprehensive income or directly in equity. In this case, the tax is also recognized in other comprehensive income or directly in equity, respectively.

The Company's management periodically evaluates positions taken in tax returns with respect to situations in which applicable tax regulation is subject to interpretation. It establishes provisions where appropriate on the basis of amounts expected to be paid to the tax authorities.

Deferred income tax is recognized, using the liability method, on temporary differences arising between the tax basis of assets and liabilities and their carrying amounts in the consolidated financial statements. However, the deferred income tax is not accounted for if it arises from initial recognition of an asset or liability in a transaction other than a reorganization that at the time of the transaction affects neither accounting nor taxable profit and loss. Deferred income tax is determined using tax rates (and laws) that have been enacted or substantially enacted by the balance sheet date and are expected to apply when the related deferred income tax asset is realized or the deferred income tax liability is settled.

Deferred income tax assets are recognized only to the extent that it is probable that future taxable profit will be available against which the temporary differences can be utilized.

Deferred income tax is provided on temporary differences arising on investments in subsidiaries and associates, except where the timing of the reversal of the temporary difference is controlled by the Company and it is probable that the temporary difference will not reverse in the foreseeable future.

Deferred income tax assets and liabilities are offset when there is a legally enforceable right to offset current tax assets against current tax liabilities and when the deferred income taxes assets and liabilities relate to income taxes levied by the same taxation authority on either the taxable entity or different taxable entities where there is an intention to settle the balances on a net basis.

2.16 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 has no legal or constructive obligation to pay further contributions if the plan does not hold sufficient assets to pay all employees the benefits relating to employee service in the current and prior periods. The contributions are recognized as employee benefit expense when they are due. Prepaid contributions are recognized as an asset to the extent that a cash refund or a reduction in the future payments is available.

(b) Termination benefits

Termination benefits are payable when employment is terminated by the Company before the normal retirement date, or whenever an employee accepts voluntary redundancy in exchange for these benefits. The Company recognizes termination benefits when it is demonstrably committed to either: terminating the employment of current employees according to a detailed formal plan without possibility of withdrawal; or providing termination benefits as a result of an offer made to encourage voluntary redundancy. Benefits falling due more than 12 months after the end of the reporting period are discounted to their present value.

(c) Bonus plans

The Company recognizes a liability and an expense for bonus plans if contractually obligated or if there is a past practice that has created a constructive obligation.

2.17 Share-Based Compensation

uniQure 2012 share option plan

The Company operates a share-based payment plan, which is an equity settled share option plan under which options have been granted in 2012 and 2013.

The fair value of the options in exchange for the services received is recognized as an expense. The total amount to be expensed over the vesting period, if any, is determined by reference to the fair value of the options granted. For the equity-settled option plan, the fair value is determined at the grant date. For share-based payments that do not vest until the employees have completed a specified period of service, uniQure recognizes the services received as the employees render service during that period. For the allocation of the expenses to be recognized, the Company treats each installment of a graded vesting award as a separate share option grant. The share options' vesting periods are as follows: 33.33% vests after one year from the initial vesting date and the remaining 66.66% vest daily on a straight-line pro rata basis over years two and three.

At each balance sheet date, the Company revises its estimates of the number of options that are expected to become exercisable. It recognizes the impact of the revision of original estimates, if any, in the income statement and a corresponding adjustment to equity.

2.18 Provisions

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

Provisions are 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 recognised as interest expense.

2.19 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 group.

License revenues

License revenues consist of upfront payments and milestone payments.

(a) Upfront payments

Revenues from non-refundable, up-front payments are initially reported as deferred revenue on the consolidated balance sheet and are recognized in the income statement as revenue over the period of the development, commercialization, collaboration or the manufacturing obligation.

(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.

2.20 Other income

uniQure's other income comprises certain subsidies that support uniQure's research efforts in defined research and development projects. These subsidies generally provide for reimbursement of approved costs incurred as defined in various grants.

2.21 Government grants

The Company receives certain government and regional grants that support its research effort in defined projects. These grants generally provide for reimbursement of approved costs incurred as defined in the respective grants. Income in respect of grants includes contributions towards the costs of research and development. Income is recognized when costs under each grant are incurred in

accordance with the terms and conditions of the grant and the collectability of the receivable is reasonably assured.

Government and regional grants relating to costs are deferred and recognized in the income statement over the period necessary to match them with the costs they are intended to compensate. When the cash in relation to recognized government or regional grants is not yet received the amount is included as a receivable on the balance sheet.

Where the grant income is directly related to the specific items of expenditure incurred, the income will be netted against such expenditure. Where the grant income is not a specific reimbursement of expenditure incurred, the Company includes such income under 'Other income' in the income statement.

Grants or investment credits may be repayable if uniQure successfully commercializes a relevant program that was funded in whole or in part by the grant or investment credit within a particular timeframe.

Prior to successful commercialization, uniQure does not make any provision for repayment.

2.22 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.

2.23 Leases

Operating leases

Leases in which a significant portion of the risks and rewards of ownership are retained by the lessor are accounted for as operating leases. Payments made under operating leases (net of any incentives received from the lessor) are charged to the income statement on a straight-line basis over the period of the lease.

Finance leases

The Company leases certain laboratory equipment and office equipment. Leases for leasehold improvements and equipment where the Company bears substantially all the risks and rewards of ownership are accounted for as finance leases. Finance leases are capitalized at the lease's commencement at the lower of the fair value of the leased property or the present value of the minimum lease payments.

Each finance lease payment is allocated between the liability and finance charges in order to achieve a constant rate on the finance balance outstanding. The finance balances, net of finance charges, are included in other long- term payables. The interest element of the finance cost is charged to the income statement over the lease period to produce a constant periodic rate of interest on the remaining balance of the liability for each period. The laboratory and office equipment acquired under finance leases are depreciated over the shorter of the useful life of the asset or the lease term.

2.24 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.

Risk management is carried out by the finance department. The finance department identifies and evaluates financial risks and hedges these risks if deemed appropriate. Since December 31, 2012 the Company has continued to strengthen the finance department which is responsible for financial risk management, through the appointment of additional senior personnel. There have been no changes in the Company's financial risk management policies, since December 31, 2012.

(a) Market Risk

(i) Currency risk

uniQure operates within the Euro area and also internationally and is exposed to foreign exchange risk arising from various currency exposures, primarily with respect to the U.S. dollar and, to a lesser extent, the British pound as the Company acquires certain materials and pays for certain licenses and other services in these two currencies.

At December 31, 2013 there was a net amount of trade payables in U.S. Dollars of €0.4 million (2012: €0.0 million) and a net trade payable in British Pounds of €0.1 million (2012: €0.2 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 an immaterial effect on the financial statements.

In the absence of significant foreign exchange exposure, management has not set up a policy to manage the foreign exchange risk against the functional currency.

As of December 31, 2013 and December 31, 2012, there would not have been a significant effect on the Company's loss due to strengthening or weakening of the functional currency against any foreign currency.

(ii) Price risk

The market prices for the provision of preclinical and clinical materials and services, as well as external contracted research may vary over time. The commercial prices of any of the Company's products or product candidates are currently uncertain. The Company is not exposed to commodity price risk.

uniQure does not hold investments classified as available-for-sale or at fair value through profit or loss; therefore uniQure is not exposed to equity securities price risk.

(iii) Cash flow and fair value interest rate risk

The Company's interest rate risk arises from short and long-term borrowings. The Company has no borrowings with variable rates and is not exposed to cash flow interest rate risk. Borrowings issued at fixed rates expose the Company to fair value interest rate risk. During 2012 the Company's borrowings were wholly denominated in Euro; in July 2013 the Company entered into an agreement with Hercules Technology Growth Capital for a $10 million denominated loan.

At December 31, 2013 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 €42,000 (2012: nil) 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 group 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 and retail customers, including outstanding receivables and committed transactions.

The Company has currently no wholesale debtors other than Chiesi. Please refer to Note 17 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.

For banks and financial institutions, only independently rated parties with a minimum rating of 'A' are accepted.

As of December 31, 2013 and December 31, 2012, the majority of uniQure's cash and cash equivalents were placed at the following banks:

	AS OF DECEMBER 31,			
	2012		2013	
(€ in thousands)	AMOUNT	CREDIT RATING	AMOUNT	CREDIT RATING
Bank				
Rabo Bank[1]	258	AA2	23,810	AA2
Van Lanschot[2]	5	A-	—	A-
Total	263		23,810	

(1) Rating is by Moody's

(2) Rating is by Fitch Ratings

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, 2013, when taken together with additional funds raised since that date, are sufficient to carry out the business plans going forward, at least until 12 months from the date of these financial statements. Prudent liquidity risk management implies maintaining sufficient cash, and planning to raise cash if and when needed, either through issue of shares or through credit facilities. Management monitors rolling forecasts of uniQure's liquidity reserve on the basis of expected cash flow.

The table below analyzes the Company's financial liabilities in relevant maturity groupings based on the length of time until the contractual maturity date, as at the balance sheet date. The amounts

disclosed in the table are the contractual undiscounted cash flows. Balances due within 12 months equal their carrying value balances as the impact of discounting is not significant.

	LESS THAN 1 YEAR	BETWEEN 1 AND 2 YEARS	BETWEEN 2 AND 5 YEARS	OVER 5 YEARS
	(€ in thousands)			
At December 31, 2012				
Borrowings (excl. finance lease liabilities)	—	—	—	—
Financial lease liabilities	151	450	—	—
Debt to related party	1,618	—	—	—
Trade and other payables	3,916	—	—	—
Total	5,685	450	—	—
At December 31, 2013				
Borrowings (excl. finance lease liabilities)	633	2,722	3,911	—
Financial lease liabilities	156	168	134	—
Debt to related party	—	—	—	—
Trade and other payables	7,445	—	—	—
Total	8,234	2,890	4,045	

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);
- 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, 2013, 2012 and 2011 financial instruments at fair value through profit and loss amounted to €3,446,000, €464,000, and nil, respectively, and comprised of movements on the fair value of the derivative elements of convertible loans, as described further in Note 14 below.

The fair value of financial instruments that are not traded in an active market (for example, over-the-counter derivatives) is determined by using valuation techniques. These valuation techniques maximize the use of observable market data where it is available and rely as little as possible on entity specific estimates. If all significant inputs required to ascertain the fair value of an instrument are observable, the instrument is included in level 2.

If one or more of the significant inputs is not based on observable market data, the instrument is included in level 3.

The carrying amount of a financial asset or financial liability is a reasonable approximation of the fair value and therefore information about the fair values of each class has not been disclosed.

	LEVEL 1	LEVEL 2	LEVEL 3	TOTAL
At December 31, 2012				
Debt to related party—embedded derivative (warrants)	—	—	132	132
Borrowings—embedded derivative (warrants)	—	—	—	—
	—	—	132	132

	LEVEL 1	LEVEL 2	LEVEL 3	TOTAL
At December 31, 2013				
Debt to related party—embedded derivative (warrants)	—	—	722	722
Borrowings—embedded derivative (warrants)	—	—	217	217
	—	—	939	939

	LEVEL 3
Opening Balance at January 1, 2013	132
Transfers to level 3	366
Movement in Equity on early conversion of the convertible loan	(3,005)
Losses recognized in Profit and Loss during the 12 months ended December 31 2013	3,446
Closing balance at December 31, 2013	939
Total losses for the period included in P&L for assets held at the end of the reporting period, under Finance expenses	3,446

Group valuation processes

The fair value of the level 3 liabilities as of December 31, 2013 have been determined using a Black-Scholes option pricing model. Key inputs include the risk-free rate, volatility, term, exercise price, and fair value of ordinary shares. The values are included within the tables presented above. Changes in the fair values are analyzed at each reporting date during the quarterly review process.

4. Critical Accounting Estimates and Judgments

The preparation of financial statements in conformity with IFRS requires the Company to make estimates and assumptions that affect the reported amounts and classifications of assets and liabilities, revenues and expenses in the consolidated financial statements. The estimates that have a significant risk of causing a material adjustment to the financial statements are utilized for share-based compensation, income taxes, research and development expenditures and borrowings. Actual results could differ materially from those estimates and assumptions.

The preparation of financial statements in conformity with IFRS also requires the Company to exercise judgment in applying the accounting policies. Critical judgments in the application of the Company's accounting policies relate to research and development expenditures, revenues and the cost of license revenues.

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.

Revenue recognition

The Company has not generated any revenues from royalties or product sales through December 31, 2013.

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

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

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

Management has concluded that the up-front payments constitute a single unit of accounting, and accordingly, the up-front payments will be recognized over the estimated remaining period of the related manufacturing technologies.

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, 2013: 170,802, with a corresponding carrying value of €939,000). The fair value of the warrants is based on the Black-Scholes model.

This model applies a number of parameters that range from observable inputs (Level 1) to un-observable inputs (Level 3). Assumptions are made on inputs such as time to maturity, the fair value per ordinary share, 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.

	Fair value per Ordinary Share	Volatility	Time to Maturity
-10%	792.000	827.000	901.000
Base Case	**939.000**	**939.000**	**939.000**
+10%	1.091.000	1.046.000	975.000

Share-based payments

In 2012 the Company introduced an equity settled share option plan. At the balance sheet date of December 31, 2013 a total of 1,691,844 options were granted and outstanding (2012: 1,606,347). This plan is accounted for in accordance with the policy as stated in Note 2.17. The option pricing model used and the inputs to that model are described in Note 12 below.

For the periods ending December 31, 2011, 2012 and 2013 the recorded expenses for share based expenses were €940,000, €1,767,000 and €2,023,000 respectively. The Company assumes all granted options held at December 31, 2013 will be held until full vesting. 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 Netherlands. Significant judgment is required in determining the use of net operating loss carry forwards and taxation of upfront and milestone payments for corporate tax purposes. There are many transactions and calculations for which the ultimate tax determination is uncertain. Where the final tax outcome of these matters is different from the amounts that were initially recorded, such differences will impact the current and deferred corporate tax assets and liabilities in the period in which such determination is made.

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 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, which is expected to occur mid 2014, the intangible asset will be carried at its cost less any accumulated amortization and any accumulated impairment losses (cost model). The estimated useful life over which the intangible will be amortized is estimated at approximately 19 years.

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

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

(c) Impairment of Assets

Assets that are subject to amortization are reviewed for impairment whenever events or changes in circumstances indicate that the carrying amount may not be recoverable. In the year ended December 31, 2013, 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 sales, 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.

The Company has determined that no impairment charge is required for the year ended December 31, 2013. 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. This conclusion was further supported by the IPO proceeds realized in February 2014 and the current market capitalization.

Based on management's expectations of revenues and gross margin from anticipated sales of Glybera by Chiesi, the management has determined that no impairment charge in respect of intangible assets relating to Glybera is necessary. These expectations are principally based on management's estimate of the market size for Glybera and the gross margin that management expects to realize.

(d) Compound Financial Instruments

Management classifies a financial instrument or its component parts on initial recognition as a financial liability, a financial asset or an equity instrument in accordance with the substance of the contractual arrangement and the definitions of a financial liability, a financial asset and an equity instrument. As described under Notes 14, we have analyzed the convertible loan issued in 2012 and concluded that both the loan and the convertible elements qualified as financial liabilities. Note 14 contains further details relating to the valuation of the convertible element.

5. Intangible Assets

	INTANGIBLE ASSETS (€ in thousands)
At January 1, 2012	
Cost	2,725
Accumulated amortization and impairment	—
Net book amount	2,725
Year ended December 31, 2012	
Opening net book amount	2,725
Additions	553
Amortization expense	—
Closing net book amount	3,278
At December 31, 2012	
Cost	3,728
Accumulated amortization and impairment	—
Net book amount	3,278
Year ended December 31, 2013	
Opening net book amount	3,278
Additions	4,652
Reductions	(155)
Amortization expense	—
Closing net book amount	7,775
At December 31, 2013	
Cost	7,775
Accumulated amortization and impairment	—
Net book amount	7,775

In the years presented in these financial statements, no amortization expense was recorded because the related products for which licenses have been granted have either not yet been approved for commercial sale by regulatory authorities, or uniQure lacked the financial and technical resources to be confident of completing the remaining development (and therefore such approved products are not yet available for use) and or approved products were not yet available for commercial sale. For the amount associated with Glybera amortization will start the month the first commercial sales of the approved product will be recorded.

The net book amount of uniQure's intangible assets by licensor or product, is set out below:

	December, 31	
	2012	2013
Xenon	365	765
AmpliPhi	2,352	2,197
NIH	317	1,130
UCSF	244	244
St. Jude	—	250
Salk Institute	—	4
Protein Sciences Corporation	—	77
Glybera Development Costs	—	3,108
Total	**3,278**	**7,775**

The amounts set out above arose as follows:

In June 2001, the Group obtained a sub-license from Xenon Genetics, Inc. ("Xenon"), which was approved by Xenon's licensor, The University of British Columbia. The sub-licence was initially capitalized in the amount of €140,000. Xenon granted the Group the exclusive worldwide rights to use the Xenon licensed technology and to use, manufacture, distribute and sell licensed products (as defined in the sub-license agreement). The contract provides for payment of license fees, milestone payments, and a portion of the royalties received from Chiesi, which will be payable to Xenon instead. Dependent upon the progress and success of the research and development activities and sales by the Company, future milestones are capitalized when payment is probable. In 2006, the Company paid a milestone of €70,000 that was capitalized.

In December 2006, the Group acquired a sub-license from Targeted Genetics Corporation (now renamed AmpliPhi Biosciences, Inc. ("AmpliPhi"). The sub-licence was approved by AmpliPhi's licensor, The University of Pennsylvania. It is related to "AAV1 Vector" technology, and the recognized acquisition amount is €1,330,000, which was capitalized.

In 2007, the Group acquired a license from the National Institutes of Health ("NIH") in the amount of €208,000 for the production of adeno-associated virus vectors.

In 2008, the Company paid and capitalized a milestone payment of €357,000 to AmpliPhi under the above license.

In 2008, the Group capitalized licensing fees totaling €600,000 related to a license from the La Sapienza University of Rome ("La Sapienza") for technology for treatment for Duchenne Muscular Dystrophy and a license from the San Rafaelle University of Milano for technology to be used in the treatment of Factor IX Hemophilia.

In 2009, the Group accrued for and capitalized a licensing milestone of $750,000 (€511,000) to AmpliPhi which became payable on the submission of the MAA of Glybera to EMA. The payment to AmpliPhi was made in 2010.

In 2010, the Group terminated its research and license agreement with San Rafaelle University of Milano. This expense had been capitalized as an intangible asset, and accordingly this amount has been written off (€300,000).

In 2011, the Group made and capitalized a payment to the NIH in the amount of €109,000 for a license to use adeno-associated virus serotype 5.

During 2011, the Group stopped further development of its Duchenne Muscular Dystrophy program. At that time, the program had not met its scientific goals. Accordingly, the amount capitalized

(€300,000) as an intangible asset in respect of the license from La Sapienza described above has been written off.

In 2012, the Group made and capitalized a payment to AmpliPhi Biosciences Corporation of $200,000 (€154,000) in accordance with its financial obligations relating to Glybera.

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

In 2012, the Group made and capitalized a payment to the University of California at San Francisco ("UCSF") of $300,000 (€244,000) in respect of the license to certain data, know-how, and other rights relating to the program for Parkinson's disease.

On March 21, 2013, the Company secured a €10.0 million convertible loan, which provided resources to complete development of Glybera. Accordingly, from March 21, 2013, the Company has capitalized development costs relating to Glybera. Following commercial launch of the product by uniQure's commercial partner, Chiesi, which is expected to occur mid 2014, the intangible asset will be carried at its cost less any accumulated amortization and any accumulated impairment losses (cost model). The estimated useful life over which the intangible will be amortized is estimated at approximately 19 years. As at the December 31, 2013 Balance sheet date the company recorded a total of €3,108,000 related capitalized Development costs for Glybera.

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).

6. Property, Plant and Equipment

	LEASEHOLD IMPROVEMENT	CONSTRUCTION IN PROCESS	LABORATORY EQUIPMENT	OFFICE EQUIPMENT	TOTAL
			(€ in thousands)		
As of January 1, 2012					
Cost	770	—	2,939	555	4,264
Accumulated depreciation	(508)	—	(2,377)	(484)	(3,369)
Net book amount	262	—	562	71	895
Year ended December 31, 2012					
Opening net book amount	262	—	562	71	895
Additions	494	—	20	324	838
Depreciation charge	(158)	—	(312)	(78)	(548)
Closing net book amount	598	—	270	317	1,185
As of December 31, 2012					
Cost	1,264	—	2,959	879	5,102
Accumulated depreciation	(666)	—	(2,689)	(562)	(3,917)
Net book amount	598	—	270	317	1,185
Year ended December 31, 2013					
Opening net book amount	598	—	270	317	1,185
Additions	—	1,285	175	504	1,964
Depreciation charge	(185)	—	(124)	(226)	(535)
Closing net book amount	413	1,285	321	595	2,614
As of December 31, 2013					
Cost	1,264	1,285	3,134	1,383	7,066
Accumulated depreciation	(851)	—	(2,813)	(788)	(4,452)
Net book amount	413	1,285	321	595	2,614

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

Depreciation expense of €535,000 for the twelve months ended December 31, 2013 (twelve months ended December 31, 2012: €548,000, 2011: €591,000) has been charged in research and development expense.

Leasehold improvements include a net book value as of December 31, 2013 of €383,000 (2012: €520,000) where uniQure is lessee under a finance lease. A further description of financial lease contracts is set out in Note 13 below.

Following the reorganization in 2011, uniQure entered into revised rental agreements with AMC and its representatives, as a consequence of which certain parts of the premises, with a cost of €446,000 at December 31, 2012, are now accounted for under a finance lease instead of an operating lease; the assets covered by this change in contractual arrangements are included within the amount of €494,000 shown as additions to leasehold improvements for the year ended December 31, 2012.

7. Other Non-Current Assets

As of December 31, 2013, the amount represents a refundable security deposit for the Lexington, Massachusetts facility, paid in September 2013.

8. Trade and Other Receivables

	DECEMBER 31, 2012	DECEMBER 31, 2013
	(€ in thousands)	
Receivables from related parties	26	1,425
Other receivables	397	764
Prepaid Expenses	—	391
Social security and other taxes	418	402
Trade and other receivables	841	2,982

The fair value of trade and other receivables approximates their carrying value. As of December 31, 2013 and December 31, 2012, all trade and other receivables were assessed as fully recoverable. The carrying amount of the Company's trade receivables are fully denominated in Euro.

The receivables from related parties as of December 31, 2013 relate to invoiced amounts to Chiesi based on revenue recognized and expenses reimbursed of €1,402,000; (2012: nil). The remaining element of receivables from related parties relate to certain wage tax liabilities settled by AMT on behalf of senior management in connection with purchases of AMT depositary receipts in 2007; these amounts are repayable to uniQure on sale of the related depositary receipts or on the respective employee ceasing to be employed by the Company of €23,000; (2012: €26,000).

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 licence 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. Inventories

	DECEMBER 31, 2012	DECEMBER 31, 2013
	(€ in thousands)	
Raw materials	—	103
Work in Process/Intermediate Products	—	762
Inventories	—	865

Inventories as of December 31, 2013 were €865,000 (2012: € nil). The amount includes the raw materials that are to be capitalized in connection with the manufacturing of Glybera for commercial sale, which is expected to commence mid 2014. Also included in inventories are amounts assigned to work in progress and intermediate products following the initial production batches of Glybera.

10. Cash and Cash Equivalents

	DECEMBER 31,	
	2012	2013
Cash at bank and in hand	263	23,810
	263	23,810

The cash balance as of December 31, 2013 reflects the receipt of €17,000,000 in up-front payments from Chiesi (July 2013), the €14,000,000 investment in equity from Chiesi (July 2013), €10,000,000 in convertible debt financing from Coller Capital (March 2013), $10,000,000 in venture debt financing from Hercules Technology Growth Capital (June 2013) and the drawdown of the remaining advance relating to the December 2012 convertible loan agreement, amounting to €1,999,000.

Supplemental information relating to the Cash Flow Statement

The conversion of the €5,000,000 convertible loan, together with accrued interest of €320,000, amounting to €5,320,000 in aggregate represented a non-cash item as of December 31, 2012. The conversion of the €13,497,000 convertible loan, comprising an amount of €1,498,000 drawn down in December 2012 and the balance of €11,999,000 drawn down during 2013, represented a non-cash item as of December 31, 2013. Refer to Note 14 below.

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 €628,000 largely related to the purchase of fixed assets, which have not yet been paid as of December 31, 2013. (2012 and 2011: nil)

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

11. Shareholders' (Deficit)/Equity

uniQure was incorporated on January 10, 2012; therefore, the year ending December 31, 2012 is the first accounting period for the Company. As described in Note 1 above, the business combination between uniQure and the AMT Group is accounted for as a reverse acquisition and the consolidated financial statements of the AMT Business are presented as the consolidated financial statements of uniQure, with an adjustment required to reflect the capital of uniQure.

The amount recognized as issued equity interests in the consolidated financial statements is determined by the issued equity interest in AMT outstanding immediately prior to the business combination, but the equity structure (the number and type of equity interests issued) reflects the equity structure of uniQure. Accordingly the share capital and share premium accounts of AMT disclosed in its audited consolidated financial statements for prior years are restated as if uniQure ordinary shares had been issued. The exchange ratio of uniQure shares issued for AMT shares was 1-for-1, but because AMT shares had a nominal value of €0.20 and uniQure shares have a nominal

value of €0.05, the impact of this approach is to reduce the balance of the share capital reported within the previous AMT accounts and correspondingly increase the balance on the share premium account.

	NUMBER OF SHARES	AMOUNT OF AMT CAPITAL *(BASED ON SHARES OF €0.20 NOMINAL VALUE)* (€ in thousands)	AMOUNT OF UNIQURE CAPITAL *(BASED ON SHARES OF €0.05 NOMINAL VALUE)*
Share capital (ordinary shares)			
As of January 1, 2011	**4,702,445**		
Share capital		940	235
Share premium		99,136	99,841
Total		100,076	100,076
New shares issued	**47,180**		
Share capital		10	2
Share premium		98	106
Total		108	108
As of January 1, 2012	**4,749,625**		
Share capital		950	237
Share premium		99,234	99,947
Total		100,184	100,184
New shares issued prior to April 5, 2012	**1,470,588**		
Share capital		294	74
Share premium		2,206	2,426
Total		2,500	2,500
Shares in issue at April 5, 2012	**6,220,213**		
Share capital		1,244	311
Share premium		101,440	102,373
Total		102,684	102,684
New shares issued after April 5, 2012	**3,433,282**		
Share capital		n/a	172
Share premium		n/a	12,422
Total		n/a	12,594
As of December 31, 2012	**9,653,495**		
Share capital		n/a	483
Share premium		n/a	114,795
Total		n/a	115,278
New shares issued after December 31, 2012	**2,541,411**		
Share capital		n/a	127
Share premium		n/a	27,664
Total		n/a	27,791
As of December 31, 2013	**12,194,906**		
Share capital		n/a	610
Share premium		n/a	142,459
Total		n/a	143,069

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.

Following the general meeting of shareholders of uniQure on July 22, 2013, the Company's authorized share capital was increased from € 1,900,000 or 38,000,000 shares, to €2,000,000 or 40,000,000 shares through the creation of an additional €100,000 or 2,000,000 class C ordinary shares, in connection with the intended equity investment by Chiesi which took place on July 24, 2013. The authorized share capital of uniQure was as follows as of December 31, 2013:

	A	B	C	TOTAL
Number of Ordinary Shares	34,281,263	3,718,737	2,000,000	40,000,000
Value (€)	1,714,063	185,937	100,000	2,000,000

As of December 31, 2013, a total of 12,194,906 shares were issued and paid up in full at a nominal value of €0.05 per share (2012: 9,653,495 shares at €0.05 per share and 2011: 4,749,625 AMT shares at €0.20 per share prior to adjustment in accordance with IFRS 3 and restated as if they were uniQure shares at €0.05 per share). Of these, 2,541,411 are presented as being issued during the year (2012: 4,902,473 shares, 2011: 47,180 shares). The total gross payment with respect to these shares issued during the period is presented as €27,791,000 (2012: €15,094,000, 2011: €108,000).

Date	Description	Sub-class of ordinary shares	Number of shares	Share capital Amounts	Share premium Amounts	Total equity Amounts
					(€ in thousands)	
January 1, 2012	Brought forward		4,749,625	237	99,947	100,184
January 4, 2012	Investment in AMT ordinary shares		1,470,588	74	2,426	2,500
April 5, 2012	Forbion conversion of existing convertible loan plus interest	A	1,064,000	53	5,267	5,320
April 5, 2012	Forbion new equity investment	A	1,954,395	98	5,902	6,000
April 18, 2012	Gilde new equity investment	A	325,732	16	984	1,000
November–December, 2012	Employees and other persons new equity investment	B	89,155	5	269	274
December 31, 2012			9,653,495	483	114,795	115,278
January–May, 2013	Employees and other persons new equity investment	B	90,747	4	274	278
July 24, 2013	Chiesi new equity investment	C	1,109,214	55	13,945	14,000
July 26, 2013	Conversion of 2012 & 2013 convertible loans	A	1,336,331	67	13,430	13,497
November, 2013	Exercising of options	B	5,118	1	15	16
December 31, 2013			12,194,906	610	142,459	143,069

This note describes the shares issued during the period since January 1, 2012. In summary these were as follows:

- On January 4, 2012, AMT raised €2,500,000 through the issuance of 1,470,588 new shares at a price of €1.70 per share. On April 5, 2012, uniQure acquired the AMT Business, issuing 6,220,213 class B ordinary shares, represented by uniQure DRs to the AMT Shareholders as consideration. Since this transaction is accounted for as a reverse acquisition, this issue of uniQure DRs is not disclosed separately within the consolidated financial record of the business;

- On April 5, 2012, uniQure raised €6,000,000 through the issue of 1,954,395 class A ordinary shares to Forbion, at a price of €3.07 per share. On April 5, 2012, the Company issued 1,064,000 class A ordinary shares to Forbion, at a price of €5.00 per share in consideration of the conversion of the outstanding €5,000,000 in convertible loan notes, together with accrued interest of €320,000;

- On May 17, 2012, uniQure raised €1,000,000 through the issue of 325,732 class A ordinary shares to Gilde, at a price of €3.07 per share;

- In November and December 2012, pursuant to an agreement entered into in April 2012, the Company raised a total amount of €274,000 through the issuance of an aggregate of 89,155 class B ordinary shares, represented by uniQure DRs shares to employees and related parties at a price of € 3.07 per share;

- In January 2013 pursuant to an agreement entered into in April 2012, the Company raised 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;

- 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.

	NARRATIVE	CASH ITEMS	NON CASH ITEMS	TOTAL
			(€ in thousands)	
Jan 4, 2012	Investment in AMT ordinary shares	2,500	—	2,500
Apr 5, 2012	Forbion new equity investment	6,000	—	6,000
Apr 5, 2012	Forbion conversion of existing convertible loan plus interest	—	5,320	5,320
Apr 19, 2012	Gilde new equity investment	1,000	—	1,000
Nov-Dec, 2012	Employees and other persons new equity investment	274	—	274
As at Dec 31, 2012		9,774	5,320	15,094
Jan, 2013	Employees and other persons new equity investment	278	—	278
July 24, 2013	Chiesi new equity investment	14,000	—	14,000
July 26, 2013	Conversion of existing 2012 convertible loan	—	13,497	13,497
Nov 2013	Exercising of options	16	—	16
As at Dec 31, 2013		14,294	13,497	27,791

In 2012 and 2011 no new shares were issued upon the exercise of share options. In November 2013 a total of 5,118 shares were issued upon exercise of share options.

As of December 31, 2013, 7,258 shares were held by the stichting participatie AMT as treasury shares (2012 and 2011: 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, 2013 amount to €27,664,000 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. In 2011 the Company recorded an expense related to the AMT share option plan of €940,000. The accumulated expense related to the AMT share option plan (described further below) for the period up to April 5, 2012, amounting to €2,987,000, is offset against the retained losses at April 5, 2012 following the extinguishing of AMT and the AMT share option scheme, as set out in the Consolidated Statement of Changes in Equity.

The Company presented in other reserves the result of the conversion of the convertible loan to the amount of €3,005,000 (see Note 14).

In the years presented in these financial statements, the Company did not have any legal or other types of restricted reserves.

12. 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 2.17 above.

Any options that vest must be exercised by the tenth anniversary of the effective date of grant.

In 2012, 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.

On October 25, 2011, AMT announced a reorganization resulting in a reduction of the AMT Group's workforce of approximately 50% and subsequent transfer of its assets and liabilities to uniQure pursuant to the transaction entered into on April 5, 2012. Consequently, AMT's 2010 Plan was deemed to have been closed and the outstanding options thereunder cancelled. Accordingly, AMT recognized the remaining option expense for AMT 2010 Plan participants that remained with the Company following the reorganization on the basis of a reduced vesting period, and recognized the pro rata element of this charge in 2011. The consequence of this was a total option expense recognized and

accounted for within retained earnings of €259,000 for the period January 1 to April 5, 2012 (for the year ended December 31, 2011 the recognized charge amounted to: €940,000). On April 5, 2012, the AMT 2010 Plan and the outstanding options granted under it were cancelled. Accordingly, the accumulated reserve was transferred to retained earnings, as described in the Consolidated Statement of Changes in Equity above.

Both the 2012 Plan and AMT 2010 Plan qualify as equity-settled plans. Movements in the number of outstanding share options granted in 2012 and 2013, under the 2012 Plan, were as follows:

	2011		2012		2013	
	NUMBER	EXERCISE PRICE	NUMBER	EXERCISE PRICE	NUMBER	EXERCISE PRICE
Number of options outstanding as of January 1	270,830	9.75–14.6	379,640	9.75–14.6	1,606,347	3.07
Number of options granted	150,241	10.3	1,606,347	3.07	301,468	3.07–10.10
Number of options forfeited	(41,431)	10.3–14.6	(379,640)	9.75–14.6	(210,853)	3.07
Number of options exercised	—	—	—	—	(5,118)	3.07
Number of options outstanding as of December 31	379,640	9.75–14.6	1,606,347	3.07	1,691,844	3.07–10.10

Of the 1,691,844 options outstanding (2012: 1,606,347; 2011: 379,640), 773,442 options (2012 and 2011: nil) were exercisable. Options outstanding at the end of the year have the following weighted average remaining contractual life and ranges of exercise prices:

YEAR ENDED DECEMBER 31, 2013 WEIGHTED AVERAGE REMAINING CONTRACTUAL LIFE	RANGE EXERCISE PRICE IN EUR PER SHARE	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	1,691,844

YEAR ENDED DECEMBER 31, 2012 WEIGHTED AVERAGE REMAINING CONTRACTUAL LIFE	RANGE EXERCISE PRICE IN EUR PER SHARE	OPTIONS
1–5 years	—	—
6 years	—	—
7 years	—	—
8 years	—	—
9 years	3.07	1,606,347
At December 31, 2012	3.07	1,606,347

YEAR ENDED DECEMBER 31, 2011 WEIGHTED AVERAGE REMAINING CONTRACTUAL LIFE	RANGE EXERCISE PRICE IN EUR PER SHARE	OPTIONS
1 - 5 years	—	—
6 years	—	—
7 years	—	—
8 years	9.75–14.60	222,650
9 years	10.30–14.60	156,990
At December 31, 2011	9.75–14.60	379,640

The Black-Scholes option pricing model has been used to value these awards, based on the following key variables:

	2011	2012	2013
Options with change of control and service based vesting conditions	379,640	—	—
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	€ 9.75–14.85	€ 3.07–5.10	€ 5.00–13.40
Estimated fair value per option as of grant date	€ 1.95–2.97	€ 2.05–3.60	€ 3.40–12.35
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	50%	70–80%	70%
Expected Term: is the period from grant until the expected exercise date.	6–7 years	5.5–6.3 years	5.5–6.3 years
Exercise price (in €):	€9.75–14.85	€ 3.07	€ 3.07–10.10
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	2.3%	0.5–1.1%	0.4–1.2%

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 in total another 301,468 options were granted (of which 252,652 options to members of the Management Board and 10,000 options to a member of the Supervisory Board) 210,853 options (of which 140,652 options from a member of the Management Board and 37,507 options from a member of the Supervisory Board) were forfeited in 2013. In November 2013 a total of 5,118 options were exercised.

The 1,691,844 options outstanding at December 31, 2013, in total represent a further share based expense of €1.7 million to be recognized from 2014 through to 2016. In addition, in January 2014 the Company granted another 609,744 options to the management of 4D Molecular Therapeutics.

Expected option term

uniQure has considered various approaches to take into account the effects of expected early exercise whereby the length of the vesting period, the expected share price development, the expected share price volatility and the participants' employee level within the organization have been analyzed.

Based on the outcome of this analysis, uniQure management has determined to take the effects of expected early exercise into account by using an estimate of an option's expected life as an input into the Black-Scholes option pricing model. As historical data about employees' exercise behavior is not available, management's estimate is based on a weighted average expected option life for the entire participant group. The resulting expected weighted average life of the options granted is the midway between the vesting date and the contractual term of the options.

Valuation of ordinary shares

AMT shares were previously listed on Euronext Amsterdam. The initial valuation of €3.07 per uniQure share derived from the average closing price of AMT shares on each of the 5 business days immediately prior to February 17, 2012, the date of the announcement of the transaction between uniQure and AMT, which was also €3.07 per AMT share. Given that uniQure had no other business of its own, and that the consideration for purchase of the business and assets of AMT was a one-for-one share issue to AMT in respect of each AMT share then in issue, the Company believed this value was reasonable and reflected the market valuation of the business.

At the date of each grant of options subsequent to the transaction between uniQure and AMT, the fair value of the ordinary shares is determined by the Management Board and Supervisory Board, and takes into account the most recently available valuation of ordinary shares and the assessment of additional objective and subjective factors the Company believes are relevant.

Expected volatility

Prior to the transaction between uniQure and AMT on April 5, 2012, AMT was listed on the Euronext Amsterdam exchange from June 2007 through April 2012. This period has provided company-specific historical and implied volatility information. In April 2012, the weighting assigned to the company-specific historic volatility was 50%, and uniQure has also estimated the expected volatility based on the historical volatility of the publicly traded peer companies for the remaining 50% weighting. For option grants post April 2012, the volatility has been estimated solely by reference to the historical volatility of the publicly traded peer companies. This has resulted in a volatility in the range 70 - 80% in respect of the options granted in the year ended December 31, 2012, and an applied volatility of 70% in respect of the options granted in the year ended December 31, 2013.

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.

13. 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, ending 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,	
	2012	2013
Gross finance lease liabilities—minimum lease payments		
No later than 1 year	184	184
Later than 1 year and no later than 5 years	505	322
Later than 5 years	—	—
Future finance charges on finance leases	(88)	(48)
Total	601	458

The present value of finance lease liabilities is as follows:

	December 31,	
	2012	2013
No later than 1 year	151	156
Later than 1 year and no later than 5 years	450	302
Later than 5 years	—	—
Future finance charges on finance leases	—	—
Total	601	458

14. Debt to related party

December 2009 Convertible loan

On December 16, 2009, AMT entered into a convertible loan agreement with Forbion, one of its major shareholders, in respect of five-year unsecured and unsubordinated loan notes ("2009 Notes"), which had an issue price of 100% and paid an annual coupon of 5%. This loan was drawn down on December 23, 2009. During the conversion period, which started six months after the funding date (or at the earlier occurrence of a limited number of events, such as a public offer to acquire AMT) and which ended on the final maturity date, the 2009 Notes were convertible into ordinary shares of AMT at an initial conversion price of €19.55, representing a conversion premium compared to AMT's share price at the date of issue of approximately 30%. The conversion price could be adjusted in the case of certain dilutive events, including an issue of shares at a discount to the average share price over the preceding five day period. As a consequence, the private placement by AMT on October 6, 2010, resulted in such an adjustment to the conversion price of the bonds from €19.55 per share to €18.45 per share, representing a conversion premium compared to AMT's share price at this date of 54%.

On April 5, 2012 the obligations under the loan were transferred from AMT to uniQure, and were then converted into new uniQure shares at a conversion price of €5.00/share.

Further details on the accounting policy applied to the convertible loan agreement are described in paragraph 2.10 (convertible loan) above.

At December 31, 2011 the conversion price of the convertible loan was above the market price of AMT ordinary shares. In such a situation the convertible loan was not regarded as being dilutive at December 31, 2011.

The valuation methodology used for the option part employed a Black-Scholes approach on the assumption that the loan would not be converted before its maturity date.

Under IFRS 7.27, the relevant factors considered within the valuation model for the compound of the instrument are as follows:

- AMT share price of €1.83 at December 31, 2011;
- Conversion price of €18.45 at December 31, 2011;
- Expected life of the instrument of 3 years;
- Annualized volatility of AMT share price of 50%;
- Implied call price of €27.68 (being 150% of the €18.45 exercise price);
- Annual rate of quarterly dividends of 0%; and
- Discount rate—Bond yield equivalent of 0.779%.

The rate used in 2011 for discounting the financial liability represented by the loan element of the convertible in 2011 was 8.5% per annum.

On February 17, 2012, AMT announced the sale and transfer of the AMT Business to uniQure. Under the terms of the transaction, the convertible loan was transferred to uniQure and then converted at a subscription price of €5.00 per share.

December 2012 Convertible loan and amendment in March 2013

On December 17, 2012, uniQure entered into a convertible loan agreement with four of its major shareholders (Forbion, Gilde, Grupo Netco and Lupus Alpha), in respect of unsecured and unsubordinated loan notes, which have an issue price of 100% and pay an annual coupon of 8%. Of the total loan €1,498,000 was drawn down in the period to December 31, 2012 and the balance of €1,999,000 was drawn down in the period from January 1, 2013 to January 31, 2013, amounting to a total convertible loan amount of €3,497,000.

In March 2013, uniQure increased the loan by an additional €10,000,000 investment by Coller Capital. As part of the increase, the loan note terms for all loan note holders described in the annual consolidated financial statements were amended such that the final maturity date of the loan notes was extended to December 31, 2014. Additionally, the warrant entitlement was reduced to 10% of the principal amount of the loan provided to uniQure.

Following the subscription for new equity by Chiesi, on July 21, 2013 the full convertible loan 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 extinction of the convertible derivative instrument. The remaining derivative element arises from the warrants issued to the holders of the convertible loan as part of the convertible loan arrangements.

The warrants associated with the convertible loan, and which survive the conversion of the loan, are presented in the consolidated Balance Sheet as at December 31, 2013 within liabilities as an embedded derivative with a fair value of €722,000 (December 31, 2012: €132,000).

During the period ending December 31, 2013, an amount of €4,387,000 (compared with €547,000 for period ending December 31, 2012) was recorded as finance expense. This amount relates to €3,491,000 of derivative results (compared with €464,000 for the period ending December 31, 2012) and the remainder consists of interest expense in relation to the convertible note, Hercules borrowing and interest expense on the financial lease.

The elimination of the embedded derivative (convertible element) by the early conversion of the loan created €3,005,000 of Other Reserves within the Equity presentation.

15. Trade and Other Payables

	DECEMBER 31, 2012	DECEMBER 31, 2013
	(€ in thousands)	
Trade payables	2,099	3,507
Social security and other tax	152	802
Other current liabilities	1,816	3,292
Total trade and other payables	4,067	7,601

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

Other current liabilities

As of December 31, 2013 and December 31, 2012, other current liabilities consisted principally of accruals for services provided by vendors but not yet billed, reimbursements received from research and development partners for expenses which have yet to be incurred and miscellaneous liabilities.

16. Borrowings

	DECEMBER 31, 2012	DECEMBER 31, 2013
	(€ in thousands)	
Non-current		
Borrowings	—	6,292
Total non-current	—	6,292
Current		
Debt to related party—Financial liability (see Note 14)	1,366	—
Debt to related party—Embedded derivative (see Note 14)	132	722
Borrowings	—	633
Borrowings—Embedded derivative	—	217
Total current	1,498	1,572
Total	1,498	7,864

Hercules Borrowing

The presented non-current borrowings relate to the Hercules Technology Growth Capital venture debt loan facility, entered into on June 14, 2013 for a book value of €6,925,000 as of December 31, 2013, presented net of expenses for facility charges of 1.25% plus expenses related to legal counsel. The loan commitment is $10 million with an interest rate of 11.85% and a back-end fee of 3.45%, which matures over a period of 39 months from the loan closing date. The interest-only period was initially set at 9 months and was extended to 15 months on completion of the transaction with Chiesi. In addition, the loan is secured by a lien on all of the Company's assets. The venture debt loan facility is governed by certain covenants and as per December 31, 2013 the Company is in compliance with these covenants.

On the balance sheet for the period ending December 31, 2013 the book value of €6,925,000 (fair value €7,490,000) is represented by a non- current element of €6,292,000 and a current element of €633,000. The fair value of the current borrowings does not equal the carrying amount of the loan as of December 31, 2013. The fair value is based on scheduled cash flows (future interest and principal

payments) discounted using a rate of 13.5% (2012: not applicable as the loan initiated in 2013) and are within level 2 of the fair value hierarchy.

The warrant included in this loan agreement is not closely related to the host contract and therefore has been split and accounted for separately as a financial derivative measured at fair value though profit or loss. The fair value of this embedded derivative is €217,000 and is included within the Current liabilities: Borrowings—embedded derivative on the consolidated balance sheet as of December 31, 2013.

There is no exposure for the Company's borrowings to interest rate changes and contractual repricing. Interest rates are fixed until maturity.

17. Revenues and Deferred Revenues

	For the years ended		
	DECEMBER 31, 2011	DECEMBER 31, 2012	DECEMBER 31, 2013
		(€ in thousands)	
License Revenues	—	—	440
Collaboration Revenues	—	—	2,503
	—	—	2,943

	DECEMBER 31, 2012	DECEMBER 31, 2013
	(€ in thousands)	
Deferred License Revenues Current Portion	—	1,279
Deferred License Revenues	—	15,679
	—	16,958

During the period ending December 31, 2013, an amount of €440,000 (period endings December 31, 2012 and December 31, 2011: €nil) was recognized as license revenues. This amount relates to the recognition of the up-front payments received from Chiesi. During the period ending December 31, 2013, an amount of €2,503,000 (periods ending December 31, 2012 and December 31, 2011: €nil) was recognized as collaboration revenues. This amount related to certain approved activities the Company was able to recharge and reimbursements of expenses under its Co-Development Agreement with Chiesi in respect of its Hemophilia B program.

Upon signing of the Commercialization Agreement and the Co-Development and Commercialization Agreement with Chiesi on April 29, 2013, the Company received €17,000,000 as a non-refundable upfront payment. Based on an assessment performed to the Company, the €17,000,000 will be amortized on a straight-line basis, and presented as license revenues, over a period from July 2013 through September 2032: the date of expiration of the last intellectual property protection related to the manufacturing process. The Company determined that the €17,000,000 of up-front payments received from Chiesi constituted a single unit of accounting. The up-front payments related to licenses and reimbursement of past development costs for Glybera and hemophilia B as follows:

1) €2,000,000—Reimbursement of past development costs related to Glybera. Continuing performance obligation: maintaining the market authorization for Glybera (including the post-approval commitment to conduct the Phase IV study);

2) €5,000,000—for past development costs related to hemophilia B. Continuing performance obligation: complete the Co-Development program and file for Marketing Authorization in the European Union;

3) €10,000,000—for having set up an EMA approved manufacturing/production facility. Continuing performance obligation: supply of commercial product to Chiesi.

Although the Company believes that the different elements have different cost levels, the Company is not able to properly estimate the respective fair values of the various elements. Therefore, the Company has concluded that the three deliverables within the arrangement are linked in such a way that the commercial effect cannot be understood without reference to the series of transactions as a whole. Therefore, the individual performance obligations were combined as a single unit of accounting and the total arrangement consideration will be recognized over the estimated life of the agreements under which the continuing performance obligations exist.

The elements described above are based on the current assumption that hemophilia B is anticipated to receive regulatory approval in late 2018, and that the commercial launch is within 3 months following approval. Based on the above, best estimate of the anticipated duration of the agreements is in line with the expiration term of the patent for manufacturing of commercial product which is 19 years. Based on the aforementioned facts, the Company has deferred the revenue and will recognize the €17,000,000 of up-front payments as license revenue on a straight-line basis over 19 years.

For the period ending December 31, 2013, the Company recognized an expense, under Costs of goods sold, in relation to its obligation to repay to the Dutch Government a portion of a grant received between 2001 and 2005 in connection with the development of Glybera; the amount was calculated as an agreed 40% of the upfront payment received in relation to Glybera. See a further description under Note 28, Contingent Liabilities.

Collaboration revenues from contracts, typically from delivering research and development services, relate to the agreements, and is recognized on the basis of labor hours delivered at the Agreements' full time employee rate.

Cost reimbursements to which the Company is entitled to under agreements are also recognized as collaboration revenues in the income statement in the same quarter of the recorded cost they intend to compensate, except for reimbursement of certain expenses incurred in the periods prior to the completion of the Chiesi agreements (on June 30, 2013); such revenues are recognized at the moment that Chiesi incurred the obligation to reimburse them, i.e. on June 30, 2013. When the reimbursable costs are not yet invoiced these amounts are included as a component of trade and other receivables on the balance sheet.

18. 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 €585,000 in 2013 (2012 €649,000; 2011: €2,192,000) and relates to grants received and rebates on payroll taxes.

Grant income was reduced in 2013 as this income includes an element of rebate on payroll taxes and in 2013 the levels of rebate were reduced further.

The Other Losses line represents the currency effect from regular operations whereas the currency risk associated with borrowings is presented under Finance Income or Expense.

19. Expenses by Category

Research and development costs amounted to €13,182,000, € 10,231,000 and €15,500,00 in 2013, 2012 and 2011, respectively, and consist of allocated employee costs, Good Manufacturing Practices ("GMP") facility costs, clinical development costs, collaboration costs, license costs, the costs of laboratory consumables and allocated depreciation costs. General and administrative costs amounted to €

11,628,000, €4,564,000 and €3,807,000 in 2013, 2012 and 2011, respectively, and consist of allocated employee costs, office costs, consultancy costs and administrative costs. Research and development costs and general administrative costs included the following costs by function:

	For the years ended December 31,		
	2011	2012	2013
Employee benefit expenses	8,493	8,350	11,904
Laboratory and development expenses	4,854	2,065	3,404
Legal and advisory expenses	2,416	1,622	5,001
Office and housing expenses	1,420	1,197	1,592
Patents and licenses	853	619	835
Other operating expenses	683	394	1,539
Depreciation expenses (See Note 6)	590	548	535
Other losses—net (exchange differences)	26	45	453
	19,334	14,840	25,263

20. Research and development expenses

Research and development expenses increased from €10,231,000 in the period ending December 31, 2012 to €13,182,000 in the period ending 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, (for the period ending December 31, 2011: €15,500,000).

21. General and administrative expenses

General and administrative expenses increased from €4,564,000 for the period ending December 31, 2012 to € 11,628,000 for the period ending December 31, 2013. The increase is primarily due to expenses related to consultants (commercial, operations and administrative) and professional fees, (for the period ending December 31, 2011: €3,807,000).

22. Other Comprehensive Income

For the period ending December 31, 2013 the amount shown as €12,000 represents the foreign currency translation arising from the U.S. subsidiary, which was established in 2013 (for the period ending December 31, 2012 and 2011: €nil).

23. Employee Benefit Expense

Wages and salaries in 2011 included termination expenses amounting to €228,000 incurred in respect of the redundancies of certain staff pursuant to the Company's restructuring. In 2012 the company recorded no termination expense and in 2013 a total of €157,000 was recorded.

	For the years ended December 31,		
	2011	2012	2013
Wages and salaries	5,499	4,553	5,012
Social security costs	502	361	377
Share options and depository receipts granted to directors and employees (See Note 12)	940	1,767	2,023
Pension costs—defined contribution plans	400	303	415
Other employee expenses	1,152	1,366	4,077
	8,492	8,350	11,904
Number of employees at the end of the period	85	67	87

24. Finance Income and Expense

	For the years ended December 31,		
	2011	2012	2013
Finance income:			
Interest income current accounts	70	22	58
Derivative result	207	—	44
	277	22	102
Finance expense:			
Bank borrowings—overdrafts and other debt	(42)	—	—
Derivative result arising on early conversion of the loan	—	(464)	(1,333)
Derivative result	—	—	(2,158)
Loan from related party	(379)	(63)	(691)
Venture Debt Facility	—	—	(165)
Finance leases	(14)	(20)	(40)
	(435)	(547)	(4,387)
Finance costs—net	(158)	(525)	(4,285)

25. Income Tax Expense

	For the years ended December 31,		
	2011	2012	2013
Current tax	—	—	—
Deferred tax	—	—	—
Profit/(loss) before tax	(17,300)	(14,716)	(26,820)
Expenses not deductible for tax purposes	741	2,268	2,161
Tax losses for which no deferred income tax asset was recognized	(16,559)	(12,448)	(24,659)
Tax charge	—	—	—

No tax charges or liabilities were incurred in the years 2013, 2012 and 2011 since the Company was in a loss-making position. No deferred tax asset has been recognized in respect of carry-forward losses.

Under Dutch income tax law a tax loss carry-forward is subject to a time limitation of nine years. Losses incurred in the years up to 2004 can still be offset against profits up to and including 2013. In connection with the transfer of the AMT Business from AMT to uniQure, uniQure has discussed with Belastingdienst, the Dutch tax authorities, the transfer of all accumulated tax losses that relate to the AMT Business, excluding tax losses relating specifically to the activities of the AMT legal entity.

In order to promote innovative technology development activities and investments in new technologies, a corporate income tax incentive has been introduced in Dutch tax law called the Innovations Box. For the qualifying profits, the company effectively owes only 5% income tax (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.

uniQure has recognized the full amount of its losses in the year in which they were incurred. As noted above, these losses are available for use within nine years of being incurred. The total amount of tax losses carried forward was €130,877,000 as of December 31, 2013 (2012: €106,274,000).

The expiration dates of these losses, is summarized in the following table. In the year ended December 31, 2013, the amount of unused tax losses that expired was €56,000 (2012: €nil and 2011: €644,000).

(€ in thousands)	2014	2015	2016	2017	2018	2019	2020	2021	2022
Loss expiring	1,336	1,838	3,310	35,633	16,735	18,359	16,559	12,448	24,659

26. 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,		
	2011	2012	2013
	(€ in thousands, except for per share data)		
Loss attributable to equity holders of the Company	(17,300)	(14,716)	(26,820)
Weighted average number of ordinary shares outstanding ('000)	4,709	8,637	10,796
Basic loss per share	(3.65)	(1.70)	(2.48)

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.

27. Dividends per Share

The Company did not declare dividends for the years ended December 31, 2013, 2012 and 2011.

28. 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, 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.

The lease expenditure charged to the income statement for operating leases amounts to €542,000 in the year ended December 31, 2013 (2012: €542,000). The future aggregate minimum lease payments under non-cancellable operating leases are as follows:

	For the years ended December 31,		
	2011	2012	2013
No later than 1 year	435	542	1,243
Later than 1 year and no later than 5 years	1,632	1,627	6,053
Later than 5 years		—	7,927
	2,067	2,169	15,223

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 an operating lease will be recognized as an expense on a straight line basis over the full duration of the lease, (for a total of $7,259,000 (€5,273,000)) taking into account the Lease Incentives as received from the landlord; This resulted in a monthly expense of $91,950 (€66,795); for the period ending December 31, 2013 the company accounted for an related expense of $183,900 (€134,416). As of December 31, 2013 the Company recorded a deferred rent of €680,000 ($936,000).

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:

	For the years ended December 31,		
	2011	2012	2013
No later than 1 year	343	277	327
Later than 1 year and no later than 5 years	—	—	—
Later than 5 years	—	—	—
	343	277	327

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, 2013 was €5,508,000 (2012: € 5,979,000), 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 ending December 31, 2013 the Company recognized an amount of €800,000 as a charge in the consolidated statement of comprehensive income within Costs of goods sold. This amount was paid to the Dutch Government in September 2013 and was calculated as 40% of the upfront amount received specifically related to Glybera.

Historically, the Company also received a "Technisch ontwikkelingsproject" (TOP) (or technical development project) grant from the Dutch government amounting to €130,000 on a project that was terminated. If the Company realizes income from the sale of assets developed under that grant, repayment clauses will apply. The Company has not recorded any liability to repay amounts in respect of this grant within these financial statements.

On January 5, 2010, the Company was awarded an investment credit (innovatiekrediet) from the Dutch government (Ministry of Economic Affairs—Agentschap.nl) in respect of the Company's program for Duchenne Muscular Dystrophy. The credit covers 35% of the costs incurred in respect of the program up to a maximum of €4,000,000. The credit includes a repayment clause dependent on the technical success of this program (which is expected to be demonstrated if the product can be successfully commercialized). The credit is interest-bearing at a rate of 11.4% per annum. To date, the Company has received €729,000 under this investment credit, and as of December 31, 2013, the total amount of the liability was € 1,063,000, representing the amount of the original advance together with accrued interest (2012: €956,000). The credit was to be repaid after the funded part of the program was completed in 2013, out of a percentage of revenues derived from the sales resulting from the Company's Duchenne Muscular Dystrophy program. The assets which are financed by means of the investment credit are subject to a right of pledge for the benefit of the Dutch Ministry of Economic

Affairs. The project has been terminated following failure to achieve the scientific goals and the Company does not anticipate that any amounts will be realized from this project and consequently there will not be any obligation to repay any of these amounts.

Other contingent liabilities

On October 22, 2013, the Company received a demand letter from Extera Partners, a consulting firm based in Boston, Massachusetts, regarding certain fees alleged to be owed by the Company in respect of consulting services provided in connection with the Company's collaboration agreements with Chiesi, under an engagement which expired on December 31, 2012. The total amount claimed by Extera Partners for present and future fees allegedly due as a result of the Company's collaboration agreements with Chiesi, which were entered into in the second quarter of 2013, is said to be in the order to €7,000,000 to €8,000,000; the engagement letter with Extera contained a cap limiting the maximum payment to €5,000,000.

The Company has reviewed the demand with counsel and believes that the claim is without merit, and consequently it is not expected to have financial consequences for the Company. (see also Note 31)

29. Related-Party Transactions

In the period ending December 31, 2013 and 2012, the Management Board received regular salaries and contributions to post-employment schemes. Additionally, selected members of the Supervisory Board received compensation for their services in the form of cash compensation.

Funds affiliated with Forbion Capital partners have a material interest in the Company. In addition, Professor Sander van Deventer and Mr. Sander Slootweg, who were appointed as members of the Supervisory Board of uniQure on April 5, 2012, are each partners of Forbion. Based on the information above, Forbion is a related party of uniQure.

Funds affiliated with Gilde Healthcare have a material interest in the Company. In addition, Mr. Edwin de Graaf, who was appointed as a member of the Supervisory Board of uniQure on April 5, 2012, 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 Grupo Netco and Lupus Alpha also have material interests in the company. Chiesi became a related party following the the commercial and investment agreements concluded with the Company on June 30, 2013, and Coller Capital became a related party following the conversion of the convertible loan in July 2013.

Transactions

The related parties identified above participated in the following transactions during the periods ended December 31, 2013 and December 31, 2012.

The 2009 convertible loan from Forbion accrued interest of 5% (a finance charge of €70,000), during the period from January 1, 2012 until its conversion on April 5, 2012 The 2012 convertible loan from Forbion, Gilde, Lupus Alpha, Grupo Netco and affiliates, and Coller Capital, as amended in March 2013, generated in the period ending 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 ending December 31, 2013, the Company received various payments from Chiesi comprising a subscription for ordinary C shares of €14,000,000 and up-front commercial payments of €16,875,000 (after deduction of Italian withholding tax). In addition, the Company received funds from Chiesi for issued invoices totaling €1,222,000.

As of December 31, 2013 the Company had a receivable outstanding with Chiesi for €1,402,000

30. Key Management Compensation

The aggregate remuneration of the Supervisory Directors amounted to €400,000 in 2013 (2012: €255,000; 2011: €174,000) as follows:

YEAR ENDED DECEMBER 31, 2013	SALARY	BONUS	SHARE-BASED PAYMENTS(1)	PENSIONS	ADVISOR'S FEE	2013 TOTAL	2012 TOTAL	2011 TOTAL
				(€ in thousands)				
Ferdinand Verdonck	—	—	244	—	37	281	43	37
Sander van Deventer(2)	—	—	—	—	—	—	8	56
Joseph Feczko	—	—	30	—	28	58	69	27
Edwin de Graaf(3)	—	—	—	—		—	—	—
Francois Meyer	—	—	30	—	28	58	69	27
Sander Slootweg(3)	—	—	—	—	—	—	—	—
Philippe Van Holle(4)	—	—	(40)	—	—	(40)	66	27
Paula Soteropoulos(5)	—	—	32	—	11	43	—	—
Robert Coffin(6)	—	—	—	—	—	—	—	—
Total	—	—	296	—	104	400	255	174

(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.

(3) Appointed April 5, 2012; Messrs de Graaf and Slootweg receive no remuneration. Mr de Graaf resigned on November 8, 2013

(4) Resigned January 1, 2013

(5) Appointed June 5, 2013

(6) Appointed November 18, 2013 and resigned December 10, 2013

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	SHORT TERM EMPLOYEE BENEFITS(1)	SHARE-BASED PAYMENTS(2)	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	1,848	1,250	169	—	—	3,267

(1) In addition the Company recognized in 2013 an expense of €55,000 (2012: €20,000, 2011: nil) in respect to the Dutch Crisis Tax levy.

(2) The share-based payment reflects the value of options expensed during the year together with a charge for the period to April 5, 2012 in respect of options granted by AMT.

The total remuneration (excluding share-based payments) paid to or for the benefit of members of the Management Board and Senior Management in 2013 amounted to approximately €2,017,000 (2012: €1,517,000).

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

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

The table below sets out a breakdown in the remuneration in 2011 of the members of the Management Board and Senior Management:

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

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

Shares and Share Options Held by Key Management

Options

	NUMBER OF OPTIONS AT JANUARY 1, 2013	OPTIONS GRANTED DURING THE YEAR	OPTIONS LAPSED/EXPIRED DURING THE YEAR	NUMBER OF OPTIONS AT DECEMBER 31, 2013
Jörn Aldag	337,565	—	—	337,565
Piers Morgan	140,652	—	—	140,652
Senior Management	562,608	252,652	(140,652)	674,608
Total	1,040,825	252,652	(140,652)	1,115,825

Depositary receipts

	NUMBER OF DEPOSITARY RECEIPTS FOR SHARES(1)
Jörn Aldag	39,389
Piers Morgan	27,805
Senior Management	16,254
Total	83,448

(1) These Depositary Receipts represent ordinary shares.

Receivables and Payables Key Management

	December 31,	
	2012	2013
Receivables from Senior Management	26	23
Total	26	23

These receivables relate to certain wage tax liabilities settled by AMT on behalf of senior management in connection with purchases of AMT depositary receipts in 2007; these amounts are repayable to uniQure on sale of the related depositary receipts or on the respective employee ceasing to be employed by the Company.

31. 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, which represents the start date of the arbitration. 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 payment to €5.0 million. On December 23, 2013 proceedings under the International Court of Arbitration formally

commenced. The Company has reviewed this claim with counsel and believes that the claim is without merit. The Company intends to vigorously defend against it.

32. Events after the balance sheet date

Since December 31, 2013 uniQure has entered into certain material agreements. These agreements do not have a material impact on the results or financial position of uniQure for the period covered by these consolidated financial statements, but are expected to have a material impact in future financial periods.

In January 2014, the Company entered into a collaboration and license agreement with 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 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 the next 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 the next 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 $91.8 million (€67.3 million) and net proceeds of $85.4 million (€62.6 million) after commissions but before expenses. On such date, the Company also reclassified its class A, B and C ordinary shares as ordinary shares.

No other events occurred after the balance sheet date that would have a material impact on the result or financial position uniQure.

True copy of deed executed on 10 February 2014

NOTE ABOUT TRANSLATION:

This document is an English translation of a document prepared in Dutch. In preparing this document, an attempt has been made to translate as literally as possible without jeopardizing the overall continuity of the text. Inevitably, however, differences may occur in translation and if they do, the Dutch text will govern by law.

In this translation, Dutch legal concepts are expressed in English terms and not in their original Dutch terms. The concepts concerned may not be identical to concepts described by the English terms as such terms may be understood under the laws of other jurisdictions.

CONVERSION AND AMENDMENT OF THE ARTICLES OF ASSOCIATION UNIQURE

Today, the tenth day of February two thousand fourteen

appeared before me, Cornelia Holdinga, civil law notary in Amsterdam, the Netherlands:

mr. Hajo Bart Hendrik Kraak, office address Holdinga Matthijssen Kraak, Diepenbrockstraat 54, 1077 WB Amsterdam, born in Tandjung Pandan, Indonesia on the twenty-fifth day of March nineteen hundred fifty.

The person appearing has declared that:

- the extraordinary general meeting of **uniQure B.V.**, a private company with limited liability, having its official seat at Amsterdam, the Netherlands, with address at Meibergdreef 61, 1105 BA Amsterdam Zuidoost, the Netherlands, registered at the Dutch Trade Register with number 54385229, hereinafter referred to as: the "**Company**", held on the twentieth day of January two thousand fourteen, in conjunction with the extraordinary general meeting of the Company held on the twenty-seventh day of January two thousand fourteen, resolved to convert the Company into a public company with limited liability ("*naamloze vennootschap*") and to amend the articles of association of the Company in its entirety as stated hereinafter as well as to authorize the person appearing to execute this deed of conversion and amendment of the articles of association, as appears from a copy of an extract from the minutes of the general meetings which will be attached to this deed as Annex I and II;

- the articles of association of the company were for the last time amended by deed executed on the thirty-first day of January two thousand fourteen the before me, civil law notary.

Subsequently the appearer declared to convert the Company into a public company with limited liability ("*naamloze vennootschap*") and to amend the articles of association of the Company in its entirety to read as follows:

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1. DEFINITIONS.

In the articles of association the following terms shall have the meaning as defined below:

- **Annual Accounts**: the annual accounts referred to in section 2:361 DCC;
- **Annual Report**: the annual report referred to in section 2:391 DCC;
- **Annual Statement of Accounts**: the Annual Accounts and, if applicable, the Annual Report as well as the additional information referred to in section 2:392 DCC;
- **Company**: the public limited company which organisation is laid down in these articles of association;
- **DCC**: the Dutch Civil Code;
- **General Meeting**: the corporate body that consists of Shareholders entitled to vote and all other persons entitled to vote / the meeting in which Shareholders and all other persons entitled to attend general meetings assemble;
- **Management Board**: the corporate body entrusted with the management of the Company;
- **Managing Director**: a member of the Management Board;
- **Meeting Rights**: the right to, either in person or by proxy authorised in writing, attend the General Meeting and to address such meeting;
- **Persons entitled to attend General Meetings**: Shareholders as well as holders of a right of use and enjoyment (*vruchtgebruik*) and holders of a right of pledge with Meeting Rights;
- **Persons entitled to vote**: Shareholders with voting rights as well as holders of a right of use and enjoyment (*vruchtgebruik*) and holders of a right of pledge with voting rights;
- **Share**: a share in the share capital of the Company;
- **Shareholder**: a holder of a Share;

· **Subsidiary**: a subsidiary as referred to in section 2:24a DCC;

· **Supervisory Board**: the corporate body entrusted with the statutory supervision of the policies of the Management Board and the other responsibilities imposed on the supervisory board by the law and these articles of association;

· **Supervisory Director**: a member of the Supervisory Board.

2. NAME. CORPORATE SEAT.

2.1. The name of the Company is: uniQure N.V.

Its corporate seat is in Amsterdam, the Netherlands, and it may establish branch offices elsewhere.

2.2. **Objects.**

The objects of the Company are:

(a) to research, develop, produce and commercialise products, services and technology in the (bio-)pharmaceutical sphere;

(b) to incorporate, participate in, conduct the management of and take any other financial interest in other companies and enterprises;

(c) to render administrative, technical, financial, economic or managerial services to other companies, persons or enterprises;

(d) to acquire, dispose of manage and exploit real and personal property, including patents, marks, licenses, permits and other intellectual property rights;

(e) to borrow and/or lend moneys, act as surety or guarantor in any other manner, and bind itself jointly and severally or otherwise in addition to or on behalf of others,

the foregoing, whether or not in collaboration with third parties, and inclusive of the performance and promotion of all activities which directly and indirectly relate to those objects, all this in the broadest sense.

3. SHARE STRUCTURE.

3.1. **Authorised share capital**

3.1.1. The authorised share capital of the Company amounts to three million euro (EUR 3.000.000,00) and is divided into sixty million (60,000,000) shares, each with a nominal value of five cent (€ 0.05).

3.1.2. The Shares shall be in registered form and shall be consecutively numbered from 1 onwards.

3.1.3. No share certificates shall be issued.

3.2. **Issue of Shares.**

3.2.1. Shares shall be issued pursuant to a resolution of the Management Board, subject to the approval of the Supervisory Board, if by resolution of the General Meeting the Management Board has been authorised for a specific period not exceeding five (5) years to issue Shares. The resolution granting the aforesaid authorisation must determine the number and class of the Shares that may be issued. The authorisation may from time to time be extended for a period not exceeding five (5)

years. Unless otherwise stipulated at its grant, the authorisation cannot be withdrawn.

3.2.2. If and insofar as an authorisation as referred to in article 3.2.1 is not in force, the General Meeting shall have the power, upon the proposal of the Management Board - which proposal must be approved by the Supervisory Board - to resolve to issue Shares.

3.2.3. Article 3.2.1 and 3.2.2 shall equally apply to a grant of rights to subscribe for Shares, but shall not apply to an issue of Shares to a person who exercises a previously acquired right to subscribe for Shares.

3.2.4. Save for the provisions of section 2:80 DCC, the issue price may not be below nominal value of the Shares.

3.2.5. Shares shall be issued by deed in accordance with the provisions of sections 2:86c and 2:96 DCC.

3.3. **Payment for Shares.**

3.3.1. Shares may only be issued against payment in full of the amount at which such Shares are issued and with due observance of the provisions of sections 2:80a and 2:80b DCC.

3.3.2. Payment must be made in cash, unless an alternative contribution has been agreed. Payment other than in cash is made with due observance of the provisions of section 2:94b DCC.

3.3.3. Payment in cash may be made in a foreign currency if the Company agrees to this. In that case, the payment obligation shall be fulfilled for the amount up to which the amount paid up can be freely exchanged into euro. This rate of exchange shall be determined by the rate of exchange prevailing on the day of payment or, after application of the provisions of the next sentence, on the day referred to there. The Company may demand payment at the rate of exchange prevailing on a specific day within two (2) months prior to the last day on which payment must have been made, provided that the Shares shall be included on the official list of any stock exchange immediately following the issue.

3.3.4. The Company may grant loans for the purpose of a subscription for or an acquisition of Shares in its share capital subject to any applicable statutory provisions.

3.3.5. The Management Board may perform legal acts as referred to in section 2:94 DCC without the prior approval of the General Meeting.

3.4. **Pre-emptive rights.**

3.4.1. Upon the issue of Shares, each Shareholder shall have a pre-emptive right to acquire such newly issued Shares in proportion to the aggregate

amount of his Shares, it being understood that this pre-emptive right shall not apply to:

(a) any issue of Shares to employees of the Company or employees of a group Company;

(b) Shares which are issued against payment in kind.

3.4.2. Pre-emptive rights may be limited or excluded by resolution of the General Meeting upon proposal of the Management Board. The Management Board, subject to approval of the Supervisory Board, shall have the power to resolve upon the limitation or exclusion of the pre-emptive right, if and to the extent the Management Board has been designated by the General Meeting. Such designation shall only be valid for a specific period of not more than five (5) years and may from time to time be extended with a period of not more than five (5) years. Unless provided otherwise in the designation, the designation cannot be cancelled.

A resolution of the General Meeting to limit or exclude the pre-emptive rights as well as a resolution to designate the Management Board as referred to in this article 3.4.2 requires a two thirds majority of the votes cast if less than half the issued share capital is represented at a meeting.

3.4.3. Without prejudice to section 2:96a DCC, the General Meeting or the Management Board, as the case may be, shall, when adopting a resolution to issue Shares, determine the manner in which and the period within which such pre-emptive rights may be exercised.

3.4.4. The Company shall announce the issue with pre-emptive rights and the period within which such rights can be exercised in such manner as shall be prescribed by applicable law and applicable stock exchange regulations, including, but not limited to, an announcement published by electronic means of communication.

3.4.5. This article 3.4 shall equally apply to a grant of rights to subscribe for Shares, but shall not apply to an issue of Shares to a person who exercises a previously acquired right to subscribe for Shares.

3.5. **Depositary receipts for shares**

The Company is not authorised to cooperate in the issue of depositary receipts for Shares.

4. OWN SHARES. CAPITAL REDUCTION.

4.1. **Acquisition of Shares.**

4.1.1. Subject to authorisation by the General Meeting, the Management Board, subject to the approval of the Supervisory Board and with due observance of the applicable relevant statutory provisions, may resolve on the acquisition by the Company of fully paid-up Shares. Such

authorisation shall only be valid for a specific period of not more than eighteen (18) months and may from time to time be extended with a period of not more than eighteen (18) months. Acquisition by the Company of non-paid up Shares is null and void.

4.1.2. The authorisation of the General Meeting as referred to in article 4.1.1 shall not be required if the Company acquires fully paid-up Shares for the purpose of transferring such Shares, by virtue of an applicable employee stock purchase plan, to persons employed by the Company or by a group Company, provided such Shares are quoted on the official list of any stock exchange.

4.2. **Capital reduction.**

4.2.1. With due observance of the statutory requirements the General Meeting may resolve to reduce the issued share capital by (i) reducing the nominal value of Shares by amending the articles of association, or (ii) cancelling:

(a) Shares in its own share capital which the Company holds itself in the Company's share capital, or

(b) all issued Shares against repayment of the amount paid-up on those Shares;

4.2.2. Partial repayment on Shares pursuant to a resolution to reduce their nominal value will be made proportionally.

5. TRANSFER.

5.1. **Form of transfer of Shares.**

5.1.1. The transfer of a Share shall require a deed executed for that purpose and, save in the event that the Company itself is a party to the transaction, written acknowledgement by the Company of the transfer. The acknowledgement is to be made either in the transfer deed, or by a dated statement endorsed upon the transfer deed or upon a copy of or extract from that deed certified by a notary (*notaris*) or bailiff (*deurwaarder*), or in the manner as referred to in article 5.1.2. Service of notice of the transfer deed or of the aforesaid copy or extract upon the Company shall be the equivalent of acknowledgement as stated in this paragraph.

5.1.2. The preceding paragraph shall apply mutatis mutandis to the transfer of any limited right to a Share, provided that a pledge may also be created without acknowledgement by or service of notice upon the Company and that section 3:239 DCC applies, in which case acknowledgement by or service of notice upon the Company shall replace the announcement referred to section 3:239, subsection 3 DCC.

6. REGISTERS. PLEDGE. USE AND ENJOYMENT (*VRUCHTGEBRUIK*).

6.1. **Shareholders register.**

6.1.1. With due observance of the applicable statutory provisions in respect of registered shares, a shareholders register shall be kept by or on behalf of the Company, which register shall be regularly updated and, at the discretion of the Management Board, may, in whole or in part, be kept in more than one copy and at more than one address. Part of the shareholders register may be kept abroad in order to comply with applicable foreign statutory provisions or applicable listing rules.

6.1.2. Each Shareholder's name, his address and such further information as required by law or considered appropriate by the Management Board, shall be recorded in the shareholders register.

6.1.3. The form and the contents of the shareholders register shall be determined by the Management Board with due observance of the articles 6.1.1 and 6.1.2.

6.1.4. Upon his request a Shareholder shall be provided free of charge with written evidence of the contents of the shareholders register with regard to the Shares registered in his name, and the statement so issued may be validly signed on behalf of the Company by a person to be designated for that purpose by the Management Board.

6.1.5. The provisions of the articles 6.1.3 and article 6.1.4 shall equally apply to persons who hold a right of use and enjoyment (*vruchtgebruik*) or a right of pledge on one or more Shares.

6.2. **Joint holding.**

If through any cause whatsoever one or more Shares are jointly held by two or more persons, such persons may jointly exercise the rights arising from those Shares, provided that these persons be represented for that purpose by one from their midst or by a third party authorised by them for that purpose by a written power of attorney.

The Management Board may, whether or not subject to certain conditions, grant an exemption for the provision of the previous sentence.

6.3. **Right of pledge.**

6.3.1. Shares may be encumbered with a pledge as security for a debt.

6.3.2. If a Share is encumbered with a pledge, the voting right attached to that Share shall vest in the Shareholder, unless at the creation of the pledge the voting right has been granted to the pledgee.

6.3.3. Shareholders who as a result of a right of pledge do not have voting rights, have Meeting Rights.

6.4. **Right of use and enjoyment (*vruchtgebruik*).**

6.4.1. Shares may be encumbered with a right of use and enjoyment.

6.4.2. If a Share is encumbered with a right of use and enjoyment, the voting right attached to that Share shall vest in the Shareholder, unless at the creation of the right of use and enjoyment the voting right has been granted to the holder of the right of use and enjoyment.

6.4.3. Shareholders who as a result of a right of use and enjoyment do not have voting rights, have Meeting Rights.

7. MANAGEMENT. SUPERVISION.

7.1. **Management. Supervision of management.**

7.1.1. The Company shall be managed by a Management Board under the supervision of a Supervisory Board. The Supervisory Board shall determine the number of Managing Directors and the number of Supervisory Directors.

7.1.2. Each Managing Director is obliged vis-a-vis the Company to perform his duties in a proper manner. These duties include all managing duties that have not been allocated to one or more other Managing Directors by law or by these articles of association. In fulfilling their tasks, the Managing Directors must be guided by the interests of the Company and its business. Each Managing Director is responsible for the Company's general course of affairs.

7.1.3. Supervision of the policies of the Management Board and of the general course of the Company's affairs and its business enterprise shall be carried out by the Supervisory Board. It shall support the Management Board with advice. In fulfilling their duties the Supervisory Directors shall serve the interests of the Company and its business enterprise. The Management Board shall in due time provide the Supervisory Board with the information it needs to carry out its duties.

7.2. **Management Board: appointment, suspension and dismissal.**

7.2.1. Managing Directors shall be appointed by the General Meeting.

7.2.2. If a Managing Director is to be appointed, the Supervisory Board shall make a binding nomination of at least the number of persons prescribed by law.

The General Meeting may at all times overrule the binding nomination by a resolution adopted by at least a two thirds majority of the votes cast, provided such majority represents more than half the issued share capital. If the General Meeting overruled the binding nomination, the Supervisory Board shall make a new nomination.

The nomination shall be included in the notice of the General Meeting at which the appointment shall be considered.

If a nomination has not been made or has not been made in due time, this shall be stated in the notice and the General Meeting shall be free to appoint a Managing Director at its discretion.

7.2.3. A resolution to appoint a Managing Director that was not nominated by the Supervisory Board may only be adopted by at least a two thirds majority of the votes cast, provided such majority represents more than half the issued share capital.

7.2.4. Managing Directors are appointed for a maximum term of four (4) years, provided that, unless a Managing Director resigns earlier, his term of appointment shall end at the close of the annual General Meeting to be held in the fourth year after the year of his appointment.

A Managing Director may be reappointed with due observance of the preceding sentence. The Supervisory Board shall draw up a retirement schedule for the Managing Directors.

7.2.5. The General Meeting shall at all times be entitled to suspend or dismiss a Managing Director. The General Meeting may only adopt a resolution to suspend or dismiss a Managing Director by at least a two thirds majority of the votes cast, provided such majority represents more than half the issued share capital, unless the proposal was made by the Supervisory Board in which case a simple majority of the votes cast is sufficient.

A second General Meeting as referred to in section 2:120, subsection 3 DCC may not be convened.

The Supervisory Board shall also at all times be entitled to suspend (but not to dismiss) a Managing Director. Within three (3) months after a suspension of a Managing Director has taken effect, a General Meeting shall be held, in which meeting a resolution must be adopted to either terminate or extend the suspension for a maximum period of another three (3) months. The suspended Managing Director shall be given the opportunity to account for his actions at that meeting.

7.2.6. If neither such resolution is adopted nor the General Meeting has resolved to dismiss the Managing Director, the suspension shall terminate after the period of suspension has expired.

In the event that one or more Managing Directors are absent or prevented from acting, the remaining Managing Director(s) shall temporarily be in charge of the management, without prejudice to the right of the Supervisory Board to replace such Managing Director for a temporary Managing Director.

In the event that all Managing Directors are, or the sole Managing Director is, absent or prevented from acting, the Supervisory Board shall temporarily be in charge of the management; the Supervisory Board shall be authorised to designate one or more temporary Managing Directors.

7.2.7. In the event that all Managing Directors are, or the sole Managing Director is, absent or prevented from acting, the Supervisory Board shall as soon as possible take the necessary measures to make a definitive

arrangement.

The term prevented from acting means:

(i) suspension;

(ii) illness;

(iii) inaccessibility,

in the events referred to under sub (ii) and (iii) without the possibility of contact between the Managing Director concerned and the Company for a period of five (5) days, unless the Supervisory Board sets a different term in the case at hand.

7.3. **Management Board: remuneration.**

7.3.1. The Company must establish a policy in respect of the remuneration of the Management Board. The remuneration policy is adopted by the General Meeting upon the proposal of the Supervisory Board.

7.3.2. The remuneration of the Management Board shall be determined by the Supervisory Board with due observance of the remuneration policy adopted by the General Meeting.

7.3.3. A proposal with respect to remuneration schemes in the form of Shares or rights to Shares is submitted by the Supervisory Board to the General Meeting for its approval.

This proposal must set out at least the maximum number of Shares or rights to Shares to be granted to members of the Management Board and the criteria for granting or amendment.

7.4. **Management Board: adoption of resolutions.**

7.4.1. If there is more than one Managing Director, the Supervisory Board can appoint one (1) of the Managing Directors as chairman of the Management Board and grant such chairman a title.

7.4.2. With due observance of these articles of association, the Management Board may adopt written rules governing its internal proceedings and providing for the division of their duties among themselves.

The adoption and amendment of the rules governing the Management Board shall be subject to the approval of the Supervisory Board without prejudice of the rights of initiative of the Supervisory Board provided for therein.

7.4.3. The Management Board shall meet whenever a Managing Director so requires. The Management Board shall adopt its resolutions by a simple majority of the votes cast. In a tie vote the chairman of the Management Board shall have a casting vote.

7.4.4. At a meeting of the Management Board, a Managing Director may only be represented by another Managing Director holding a written proxy.

7.4.5. If a Managing Director has a direct or indirect personal conflict of interest with the Company, he shall not participate in the deliberations and the decision-making process concerned in the Management Board. If as a result thereof no resolution of the Management Board can be adopted, the resolution may be adopted by the Supervisory Board.

7.4.6. The Management Board may also adopt resolutions without holding a meeting, provided such resolutions are adopted in writing or in a reproducible manner by electronic means of communication and all the Managing Directors entitled to vote have consented to adopting the resolution outside a meeting.

7.4.7. Articles 7.4.3 and 7.4.5 shall equally apply to adoption by the Management Board of resolutions without holding a meeting.

7.4.8. Without prejudice to any other applicable provisions of these articles of association, the Management Board shall require the approval of the General Meeting for resolutions of the Management Board regarding a significant change in the identity or nature of the Company or the enterprise, including in any event:

(a) the transfer of the enterprise or practically the entire enterprise to a third party;

(b) the entry into or termination of any long-lasting cooperation by the Company or a Subsidiary with any other legal person or company or as a fully liable general partner of a limited partnership or a general partnership, provided that such cooperation or the termination thereof is of significant importance to the Company; and

(c) the acquisition or disposal of a participating interest in the capital of a Company with a value of at least one-third of the sum of the assets according to the consolidated balance sheet with explanatory notes thereto according to the last adopted Annual Accounts of the Company, by the Company or a Subsidiary.

7.4.9. Without prejudice to any other applicable provisions of these articles of association, the Management Board resolutions relating to any of the following matters shall be subject to the approval of the Supervisory Board:

(a) the sale or disposition of all, or an essential part of, the assets of the Company;

(b) the issuance and acquisition of Shares and of debentures chargeable against the Company or chargeable against a limited partnership (commanditaire vennootschap), or a general partnership (vennootschap onder firma) of which the Company is the fully liable partner;

(c) the application for quotation, or withdrawal of quotation, of Shares or debt of the Company on any stock exchange;

(d) the entry into or termination of any long-term, material cooperation by the Company or a Subsidiary of the Company with another legal entity or partnership or as a fully liable general partner in a limited partnership or general partnership, if such cooperation or termination is of significant importance to the Company;

(e) the participation by the Company or a Subsidiary of the Company in the capital of another company in an amount equal to at least one fourth of the issued capital plus the reserves of the Company, as reflected in the balance sheet with explanatory notes of the Company, as well as a material change to such participation;

(f) investments requiring an amount equal to at least one fourth of the issued capital plus the reserves of the Company, as reflected in the balance sheet with explanatory notes;

(g) filing a petition for bankruptcy (faillissement) or for suspension of payments (surseance van betaling) by the Company;

(h) the termination of a significant number of the employees of the Company or a Subsidiary simultaneously or within a short period of time;

(i) a significant change in the employment conditions of the employees of the Company or of a Subsidiary; and

(j) a decrease in the issued capital of the Company.

7.4.10. The Supervisory Board may determine that a resolution that would be subjected to its approval pursuant to article 7.4.9 will not require such approval if the amount involved does not exceed a value fixed by the Supervisory Board and notified to the Management Board in writing.

7.4.11. The Supervisory Board may also require that additional actions than required under article 7.4.9 by the Management Board be subjected to the approval of the Supervisory Board. Such actions must be clearly specified to the Management Board in writing.

7.4.12. The absence of approval of the Supervisory Board does not affect the authority of the Management Board or its members to represent the Company in dealings with third parties.

7.5. **Representation.**

7.5.1. The Management Board, as well as two (2) Managing Directors acting jointly are authorised to represent the Company.

7.5.2. The Management Board may grant one or more persons, whether or not employed by the Company, the power to represent the Company

(*procuratie*) or grant the power to represent the Company on a continuing basis in a different manner.

7.6. **Supervisory Board: appointment, suspension and dismissal.**

7.6.1. Supervisory Directors shall be appointed by the General Meeting.

7.6.2. If a Supervisory Director is to be appointed, the Supervisory Board shall make a binding nomination.

The General Meeting may at all times overrule the binding nomination by a resolution adopted by at least a two thirds majority of the votes cast, provided such majority represents more than half of the issued share capital. If the General Meeting overruled the binding nomination, the Supervisory Board shall make a new nomination.

The nomination shall be included in the notice of the General Meeting at which the appointment shall be considered.

7.6.3. If a nomination has not been made or has not been made in due time, this shall be stated in the notice and the General Meeting shall be free to appoint a Supervisory Director at its discretion.

7.6.4. A resolution to appoint a Supervisory Director that was not nominated by the Supervisory Board, may only be adopted by at least a two thirds majority of the votes cast, provided such majority represents more than half of the issued share capital.

7.6.5. Supervisory Directors are appointed for a maximum term of three (3) years, provided that, unless a Supervisory Director resigns earlier, his term of appointment shall end at the close of the annual General Meeting that will be held in the third year after his appointment.

A Supervisory Director may be reappointed for a term of not more than three (3) years at a time, with due observance of the previous sentence. A Supervisory Director may be a Supervisory Director for a period not longer than twelve (12) years, which period may or may

not be interrupted, unless the General Meeting resolves otherwise. The Supervisory Board shall draw up a resignation retirement schedule for the members of the Supervisory Board.

7.6.6. The General Meeting shall at all times be entitled to suspend or dismiss a Supervisory Director. The General Meeting may only adopt a resolution to suspend or dismiss a Supervisory Director by at least a two thirds majority of the votes cast, provided such majority represents more than half the issued share capital, unless the proposal was made by the Supervisory Board in which case a simple majority of the votes cast is sufficient.

A second General Meeting as referred to in section 2:120, subsection 3 DCC may not be convened.

7.6.7. In the event that one or more Supervisory Directors are absent or prevented from acting the remaining Supervisory Director(s) shall temporarily be in charge of the supervision, without prejudice to the right of the General Meeting to replace such Supervisory Director for a temporary Supervisory Director.

In the event that one or more Supervisory Directors are absent or prevented from acting, the remaining Supervisory Directors shall as soon as possible take the necessary measures to make a definitive arrangement. In the event that all Supervisory Directors are absent or prevented from acting, the Management Board shall as soon as possible take the necessary measures to make a definitive arrangement.

The term prevented from acting means:

(i) suspension;

(ii) illness;

(iii) inaccessibility,

in the events referred to under sub (ii) and (iii) without the possibility of contact between the Supervisory Director concerned and the Company for a period of five (5) days.

7.7. **Supervisory Board: remuneration.**

The General Meeting shall determine the remuneration of Supervisory Directors. Supervisory Directors shall be reimbursed for their expenses.

7.8. **Supervisory Board: adoption of resolutions.**

7.8.1. If there is more than one (1) Supervisory Director, the Supervisory Board shall appoint one of its members as chairman. The Supervisory Board may also appoint a secretary, whether or not from among its members.

Furthermore, the Supervisory Board may appoint one or more of its members as delegate Supervisory Director to be in charge of communicating with the Management Board on a regular basis. They shall report their findings to the Supervisory Board. The offices of chairman of the Supervisory Board and delegate Supervisory Director are compatible.

7.8.2. With due observance of these articles of association, the Supervisory Board may adopt written rules governing its internal proceedings.

7.8.3. The Supervisory Board shall meet whenever a Supervisory Director so requires. The Supervisory Board shall adopt its resolutions by a simple majority of the votes cast.

In a tie vote the chairman shall have a casting vote.

7.8.4. At a meeting of the Supervisory Board, a Supervisory Director may only be represented by another Supervisory Director holding a written proxy.

7.8.5. If a Supervisory Director has a direct or indirect personal conflict of interest with the Company, he shall not participate in the deliberations and the decision-making process concerned in the Supervisory Board. If as a result thereof no resolution of the Supervisory Board can be adopted the resolution can nonetheless be adopted by the Supervisory Board. In that case each Supervisory Director shall be entitled to participate in the deliberations and the decision-making process concerned in the Supervisory Board.

7.8.6. The Supervisory Board may also adopt resolutions without holding a meeting, provided such resolutions are adopted in writing or in a reproducible manner by electronic means of communication and all Supervisory Directors entitled to vote have consented to adopting the resolution outside a meeting.

7.8.7. Articles 7.8.3 and 7.8.5 shall equally apply to adoption by the Supervisory Board of resolutions without holding a meeting.

7.8.8. The Managing Directors must, if invited to do so, attend the meetings of the Supervisory Board and they shall provide in such meetings all information required by the Supervisory Board.

7.8.9. The Supervisory Board may decide that one or more of its members shall have access to all premises of the Company and shall be authorised to examine all books, correspondence and other records and to be fully informed of all actions which have taken place, or may

decide that one or more of its members shall be authorised to exercise a portion of such powers.

7.8.10. At the expense of the Company, the Supervisory Board may obtain such advice from experts as the Supervisory Board deems desirable for the proper fulfilment of its duties.

7.9. **Indemnification Managing Directors and Supervisory Directors.**

7.9.1. Unless Dutch law provides otherwise, the following shall be reimbursed to current and former members of the Management Board or Supervisory Board:

(a) the reasonable costs of conducting a defence against claims based on acts or failures to act in the exercise of their duties or any other duties currently or previously performed by them at the Company's request;

(b) any damages or fines payable by them as a result of an act or failure to act as referred to under a;

(c) the reasonable costs of appearing in other legal proceedings in which they are involved as current or former members of the Management Board or Supervisory Board, with the exception of

proceedings primarily aimed at pursuing a claim on their own behalf.

There shall be no entitlement to reimbursement as referred to above if and to the extent that:

(d) a Dutch court or, in the event of arbitration, an arbitrator has established in a final and conclusive decision that the act or failure to act of the person concerned can be characterised as wilful (opzettelijk), intentionally reckless (bewust roekeloos) or seriously culpable (ernstig verwijtbaar) conduct, unless Dutch law provides otherwise or this would, in view of the circumstances of the case, be unacceptable according to standards of reasonableness and fairness; or

(e) the costs or financial loss of the person concerned are covered by an insurance and the insurer has paid out the costs or financial loss.

If and to the extent that it has been established by a Dutch court or, in the event of arbitration, an arbitrator in a final and conclusive decision that the person concerned is not entitled to reimbursement as referred to above, he shall immediately repay the amount reimbursed by the Company.

7.9.2. The Company may take out liability insurance for the benefit of the persons concerned.

7.9.3. The Management Board may by agreement give further implementation to the above.

8. MEETINGS.

8.1. **General Meetings.**

8.1.1. General Meetings shall be held in Amsterdam or in the municipality of Haarlemmermeer (Schiphol Airport).

8.1.2. A General Meeting shall be held once a year, no later than six (6) months after the end of the financial year of the Company.

8.1.3. The Management Board and the Supervisory Board shall provide the General Meeting with all requested information, unless this would be contrary to an overriding interest of the Company. If the Management Board or Supervisory Board invokes an overriding interest, it must give reasons.

8.2. **Extraordinary General Meetings.**

Extraordinary General Meetings shall be convened by the Management Board or Supervisory Board.

8.3. **General Meetings: notice and agenda.**

8.3.1. Notice of the General Meeting shall be given by the Management Board or Supervisory Board upon a term of at least such number of days prior to the day of the meeting as required by law, in accordance with law and the regulations of the stock exchange where the Shares in the share capital of the Company at the Company's request are officially listed.

8.3.2. The Management Board or Supervisory Board may decide that the convocation letter in respect of a person authorised to attend a General Meeting who agrees thereto, is replaced by a legible and reproducible message sent by electronic mail to the address indicated by him to the Company for such purpose.

8.3.3. The notice shall state the subjects on the agenda or shall inform the persons authorised to attend a General Meeting that they may inspect the agenda at the office of the Company and that copies thereof are obtainable at such places as are specified in the notice.

8.3.4. The agenda for the annual General Meeting shall in any case include the following items:

(a) the consideration of Annual Statement of Accounts;

(b) the adoption of the Annual Accounts;

(c) the appropriation of profits;

(d) proposals relating to the composition of the Management Board or Supervisory Board, including the filling of any vacancies in the Management Board or Supervisory Board;

(e) the proposals placed on the agenda by the Management Board or Supervisory Board together with proposals made by Shareholders in accordance with provisions of the law and the provisions of the articles of association.

8.3.5. A matter, the consideration of which has been requested in writing by one or more Shareholders, representing solely or jointly at least the percentage prescribed by law of the issued share capital, will be placed on the notice or will be announced in the same manner if the Company has received the request not later than on the date as prescribed by law.

8.3.6. The Management Board shall inform the General Meeting by means of a shareholders' circular or explanatory notes to the agenda of all facts and circumstances relevant to the proposals on the agenda.

8.4. **General Meetings: attendance of meetings.**

8.4.1. The persons who are entitled to attend the General Meeting are persons who:

(i) are a Shareholder or a person who is otherwise entitled to attend the General Meeting as per a certain date, determined by the

Management Board, such date hereinafter referred to as: the "record date";

(ii) are as such registered in a register (or one or more parts thereof) designated thereto by the Management Board, hereinafter referred to as: the "register"; and

(iii) have given notice in writing to the Company prior to a date set in the notice that they will attend a General Meeting,

regardless of who will be Shareholder at the time of the meeting. The notice will contain the name and the number of Shares the person will represent in the meeting. The provision above under (iii) concerning the notice to the Company also applies to the proxy holder of a person authorised to attend a General Meeting.

8.4.2. The Management Board may decide that Persons entitled to attend General Meetings and vote thereat may, within a period prior to the General Meeting to be set by the Management Board, which period cannot begin prior to the record date as meant in article 8.4.1, cast their votes electronically in a manner to be decided by the Management Board. Votes cast in accordance with the previous sentence are equal to votes cast at the meeting.

8.4.3. The Management Board may decide that the business transacted at a General Meeting can be taken note of by electronic means of communication.

8.4.4. The Management Board may decide that each person entitled to attend General Meetings and vote thereat may, either in person or by written proxy, vote at that meeting by electronic means of communication, provided that such person can be identified via the electronic means of communication and furthermore provided that such person can directly take note of the business transacted at the General Meeting concerned. The Management Board may attach conditions to the use of the electronic means of communication, which conditions shall be announced at the convocation of the General Meeting and shall be posted on the Company's website.

8.4.5. Managing Directors and Supervisory Directors shall have admission to the General Meetings. They shall have an advisory vote at the General Meetings.

8.4.6. Furthermore, admission shall be given to the persons whose attendance at the General Meeting is approved by the chairman of the meeting.

8.4.7. All issues concerning the admittance to the General Meeting shall be decided by the chairman of the meeting.

8.5. **General Meetings: order of the meeting, minutes.**

8.5.1. The General Meeting shall be chaired over by the chairman of the Supervisory Board. However, the chairman may charge another person

to chair the General Meeting in his place even if he himself is present at the meeting. If the chairman of the Supervisory Board is absent and he has not charged another person to chair the meeting in his place, the Supervisory Directors present at the meeting shall appoint one of them to be chairman. If no members of the Supervisory Board are present at the General Meeting, the General Meeting shall be chaired by the chairman of the Management Board, or, if the chairman of the Management Board is absent, by one of the other members of the Management Board designated for that purpose by the Management Board. The chairman shall designate the secretary.

8.5.2. The chairman of the meeting shall determine the order of proceedings at the meeting with due observance of the agenda and he may restrict the allotted speaking time or take other measures to ensure orderly progress of the meeting.

8.5.3. All issues concerning the proceedings at the meeting, shall be decided by the chairman of the meeting.

8.5.4. Minutes shall be kept of the business transacted at the meeting unless a notarial record is prepared thereof. Minutes shall be adopted and in evidence of such adoption be signed by the chairman and the secretary of the meeting concerned.

8.5.5. A certificate signed by the chairman and the secretary of the meeting confirming that the General Meeting has adopted a particular resolution, shall constitute evidence of such resolution vis-à-vis third parties.

8.6. **General Meetings: adoption of resolutions.**

8.6.1. Resolutions proposed to the General Meeting by the Management Board or Supervisory Board shall be adopted by a simple majority of the votes cast unless the law or these articles of association provide otherwise. Unless another majority of votes or quorum is required by virtue of the law, all other resolutions shall be adopted by at least a simple majority of the votes cast, provided such majority represents more than one-third of the issued share capital.

A second meeting referred to in article 2:120, subsection 3 DCC cannot be convened.

8.6.2. Each Share confers the right to cast one (1) vote at the General Meeting.

Blank votes and invalid votes shall be regarded as not having been cast.

8.6.3. No votes may be cast at the General Meeting in respect of Shares which are held by the Company or any of its Subsidiaries.

Holders of a right of use and enjoyment (*vruchtgebruik*) and pledgees of Shares which belong to the Company or its Subsidiaries shall not be excluded from the right to vote if the right of use and enjoyment or pledge was created before the Shares concerned were held by the Company or a Subsidiary of the Company and at the creation of the

right of pledge or the right of use and enjoyment, the voting rights were granted to the pledgee or holder of the right of use and enjoyment.

8.6.4. The chairman of the General Meeting determines the method of voting.

8.6.5. The ruling pronounced by the chairman of the General Meeting in respect of the outcome of any vote taken at a General Meeting shall be decisive. The same shall apply to the contents of any resolution passed.

8.6.6. Any and all disputes with regard to voting for which neither the law nor the articles of association provide shall be decided by the chairman of the General Meeting.

9. FINANCIAL YEAR. AUDITOR.

9.1. **Financial year; Annual Statement of Accounts.**

9.1.1. The financial year of the Company shall be the calendar year.

9.1.2. Annually, within the term set by law, the Management Board shall prepare Annual Accounts.

The Annual Accounts shall be accompanied by the auditor's statement referred to in article 9.2.1, if the instruction referred to in that article has been given, by the Annual Report, unless section 2:391 DCC does not apply to the Company, as well as by the other particulars to be added to those documents by virtue of applicable statutory provisions.

The Annual Accounts shall be signed by all Managing Directors and by all Supervisory Directors; if the signature of one or more of them is lacking, this shall be disclosed, stating the reasons therefor.

9.1.3. The Company shall ensure that the Annual Accounts as prepared, the Annual Report (if applicable) and the other particulars referred to in article 9.1.2 shall be made available at the office of the Company as of the date of the notice of the General Meeting at which they are to be discussed.

The Shareholders and other Persons entitled to attend General Meetings may inspect the above documents at the office of the Company and obtain a copy thereof free of charge.

9.2. **Auditor.**

9.2.1. The General Meeting shall instruct a registered accountant or another expert, as referred to in section 2:393, subsection 1 DCC, both hereinafter called: the "auditor", to audit the Annual Accounts prepared by the Management Board, in accordance with the provisions of section 2:393, subsection 3 DCC. The auditor shall report on his audit to the Management Board and shall present the results of his examination regarding the accuracy of the Annual Accounts in an auditor's statement.

9.2.2. If the General Meeting fails to give such instructions, then the Supervisory Board, or if the Supervisory Board also fails to give such instruction, the Management Board shall be so authorised.

9.2.3. The instruction given to the auditor may be revoked by the General Meeting and by the corporate body which has given such instruction; furthermore, the instruction given by the Management Board may be revoked by the Supervisory Board.

The instruction may only be revoked for good reasons with due observance of section 2:393, subsection 2 DCC.

9.2.4. The Management Board as well as the Supervisory Board may give instructions to the auditor or any other auditor at the expense of the Company.

10. PROFITS.

10.1. **Profit and loss. Distributions on Shares.**

10.1.1. The Management Board will keep a share premium reserve and profit reserve for the Shares.

10.1.2. The Company may make distributions on Shares only to the extent that its shareholders' equity exceeds the sum of the paid-up and called-up part of the capital and the reserves which must be maintained by law.

10.1.3. Distributions of profit, meaning the net earnings after taxes shown by the adopted Annual Accounts, shall be made after the adoption of the Annual Accounts from which it appears that they are permitted, without prejudice to any of the other provisions of these articles of association.

10.1.4. The Management Board may determine, subject to the approval of the Supervisory Board, that any amount out of the profit shall be added to the reserves.

10.1.5. The profit remaining after application of article 10.1.4 shall be at the disposal of the General Meeting, which may resolve to carry it to the reserves or to distribute it among the Shareholders.

10.1.6. On a proposal of the Management Board — which proposal must be approved by the Supervisory Board — the General Meeting may resolve to distribute to the Shareholders a dividend in the form of Shares in the share capital of the Company.

10.1.7. Subject to the other provisions of this article 10.1 the General Meeting may, on a proposal made by the Management Board — which proposal must be approved by the Supervisory Board — resolve to make distributions to the Shareholders to the debit of one (1) or several reserves which the Company is not prohibited from distributing by virtue of the law.

10.1.8. No dividends shall be paid on Shares held by the Company in its own share capital, unless such Shares are encumbered with a right of use and enjoyment (*vruchtgebruik*) or pledge.

10.2. **Interim distributions.**

10.2.1. The Management Board may resolve, subject to the approval of the Supervisory Board, to make interim distributions to the Shareholders if an interim statement of assets and liabilities shows that the requirement of article 10.1.2 has been met.

10.2.2. The interim statement of assets and liabilities shall relate to the condition of the assets and liabilities on a date no earlier than the first day of the third month preceding the month in which the resolution to distribute is published. It shall be prepared on the basis of generally acceptable valuation methods. The amounts to be reserved under the law and these articles of association shall be included in the statement of assets and liabilities. It shall be signed by the Managing Directors and Supervisory Directors. If the signature of one or more of them is lacking, this shall be disclosed, stating the reasons therefor.

10.2.3. Any proposal for distribution of dividend on Shares and any resolution to distribute an interim dividend on Shares shall immediately be published by the Management Board in accordance with the regulations of the stock exchange where the Shares at the Company's request are officially listed. The notification shall specify the date when and the place where the dividend shall be payable or - in the case of a proposal for distribution of dividend - is expected to be made payable.

10.2.4. Dividends shall be payable no later than thirty (30) days after the date they were declared, unless the body declaring the dividend determines a different date.

10.2.5. Dividends which have not been claimed upon the expiry of five (5) years and one (1) day after the date when they became payable shall be forfeited to the Company and shall be carried to the reserves.

10.2.6. The Management Board may determine that distributions on Shares shall be made payable either in euro or in another currency.

11. AMENDMENT OF THE ARTICLES OF ASSOCIATION; DISSOLUTION OF THE COMPANY.

11.1. A resolution to amend the articles of association or to dissolve the Company may only be adopted at the proposal of the Management Board with the prior approval of the Supervisory Board.

11.2. **Liquidation.**

11.2.1. On the dissolution of the Company, the liquidation shall be carried out by the Management Board, unless otherwise resolved by the General Meeting.

11.2.2. Pending the liquidation the provisions of these articles of association shall remain in force to the fullest extent possible.

11.2.3. The surplus assets of the Company remaining after satisfaction of its debts shall, in accordance with the provisions of section 2:23b DCC, be for the benefit of the Shareholders in proportion to the nominal value amount of the Shares held by each of them.

12. TRANSITIONAL PROVISION.

As per the date of the present conversion and amendment to the articles all the issued shares class A, class B and class C have been redesignated as ordinary shares numbered from 1 to 12,194,906 and thirty nine (39) fractions of each one cent (€ 0.01). Consequently, the issued capital as per today amounts to EUR 609,745,69.

13. ACCOUNTANTS CERTIFICATE

Pursuant to the law, the accountant certificate as required by Article 2: 18 in connection with 72 DCC is attached to the present deed, certifying that the Company's equity as per 30 September 2013, (being a date not earlier than 5 months prior to today's date) at least amounts to the issued and paid up capital of the Company.

END

This deed was executed in Amsterdam on the date first above written. The contents of this instrument were given and explained to the person appearing.

He then declared that she had timely noted and approved the contents and did not want a full reading thereof.

Thereupon, after limited reading, this instrument was signed by the person appearing and by me, civil law notary.

Exhibit 8.1

SUBSIDIARIES OF UNIQURE N.V.

Name of Subsidiary	Jurisdiction of Organization
uniQure biopharma B.V.	The Netherlands
uniQure IP B.V.	The Netherlands
uniQure Inc.	Delaware
uniQure manufacturing B.V.	The Netherlands
uniQure Assay Development B.V.	The Netherlands
uniQure Research B.V.	The Netherlands
uniQure non clinical B.V.	The Netherlands
uniQure QA B.V.	The Netherlands
uniQure process development B.V.	The Netherlands
uniQure clinical B.V.	The Netherlands

Exhibit 12.1

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)) 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) [Deliberately omitted]

(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; and

(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 25, 2014

By: /S/ JÖRN ALDAG
Name: Jörn Aldag
Title: *Chief Executive Officer*

Exhibit 12.2

Certification by the Chief Financial Officer
Pursuant to Section 302 of the Sarbanes-Oxley Act of 2002

I, Piers Morgan, 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)) 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) [Deliberately omitted]

(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; and

(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 25, 2014

By: /S/ PIERS MORGAN
Name: Piers Morgan
Title: *Chief Financial Officer*

Exhibit 13.1

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, 2013, 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 Piers Morgan, 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 25, 2014

By: /S/ JÖRN ALDAG
Name: Jörn Aldag
Title: *Chief Executive Officer*

By: /S/ PIERS MORGAN
Name: Piers Morgan
Title: *Chief Financial Officer*