# UNITED STATES SECURITIES AND EXCHANGE COMMISSION

Washington, D.C. 20549

# FORM 6-K

Report of Foreign Private Issuer Pursuant to Rule 13a-16 or 15d-16 of the Securities Exchange Act of 1934

March 3, 2015

# uniQure N.V.

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

Indicate by check mark whether the registrant files or will file annual reports under cover of Form 20-F or Form 40-F.

Form 20-F x Form 40-F o

Indicate by check mark if the registrant is submitting the Form 6-K in paper as permitted by Regulation S-T Rule 101(b)(1): o

Indicate by check mark if the registrant is submitting the Form 6-K in paper as permitted by Regulation S-T Rule 101(b)(7): o

Furnished as Exhibit 99.1 to this Report on Form 6-K is the Company's Management's Discussion and Analysis of Financial Condition and Results of Operations for the nine month periods ended September 30, 2014 and 2013.

Incorporated by reference as Exhibit 99.2 to this Report on Form 6-K are the Company's unaudited financial statements for the nine month periods ended September 30, 2014 and 2013 (previously filed as Exhibit 99.2 on a Form 6-K dated December 1, 2014).

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# SIGNATURES

Pursuant to the requirements of the Securities Exchange Act of 1934, the registrant has duly caused this report to be signed on its behalf by the undersigned, thereunto duly authorized.

# UNIQURE N.V.

By: /S/ JÖRN ALDAG

Jörn Aldag Chief Executive Officer

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# INDEX TO EXHIBITS

Number	Description
99.1	Management's Discussion and Analysis of Financial Condition and Results of Operations.
99.2	Unaudited financial statements of the Company for the nine-month periods ended September 30, 2014 and 2013 (incorporated by reference to Exhibit 99.2 of the Company's Report on Form 6-K filed with the Securities and Exchange Commission on December 1, 2014).

Date: March 3, 2015

# UNIQURE N.V.

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

The following is a discussion of our financial condition and results of operations for the nine months ended September 30, 2014. You should read the following discussion and analysis together with the unaudited condensed consolidated financial statements and related notes previously filed by the Company For additional information relating to our management's discussion and analysis of financial condition and results of operations, please see our annual report on Form 20-F for the year ended December 31, 2013 filed with the U.S. Securities and Exchange Commission on April 25, 2014.

## Overview

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

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

- using our strong technology platform to develop our own programs in three therapeutic areas, liver-based diseases, cardio/metabolic diseases, and central nervous system diseases, in which we have a competitive advantage, with the potential to significantly de-risk development programs, and reduce development cost and time to market;
- sponsoring and acquiring additional early-stage programs in these areas from other biopharmaceutical companies and academic investigators;
- enhancing and accelerating these programs through our modularized research and development platform and our experience of the EU and FDA regulatory environments for gene therapies;
- applying our industrialized manufacturing process to produce the highest-quality material for our own and our collaborators' programs, and
- collaborating with pharmaceutical companies with the necessary expertise to enhance our late-stage therapy development and maximize the value of our therapies at the commercialization stage.

We believe that our technology platform and strategic collaborations place us at the forefront of gene therapy within our chosen therapeutic areas. Our transgene delivery system is based on common, adeno-associated viruses, or AAV, which we believe are safe and effective delivery methods for efficient expression of transgenes. We have the exclusive or non-exclusive rights to natural AAV serotypes for lipoprotein lipase deficiency, or LPLD, liver and CNS applications and the capability to identify and develop synthetic AAV vectors that are designed to optimize the expression of a particular transgene in specific tissue types. We produce our AAV-based vectors in our own facilities with a proprietary, commercial-scale, consistent, and robust manufacturing process

using insect cells and baculoviruses, a common family of viruses found in invertebrates. We believe our Lexington, Massachusetts-based facility, which is currently being qualified, is one of the world's largest, most versatile, gene therapy manufacturing facilities. We believe this robust technology platform, combined with our know-how derived from achieving the first regulatory approval of a gene therapy in the European Union, provides us a significant advantage in bringing our gene therapy products to the market ahead of our competitors.

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

Our business was founded in 1998 by scientists who were investigating LPLD at the Academic Medical Center of the University of Amsterdam, or the AMC. In our early years we received funding and subsidized rent from the AMC, government grants, income for cGMP contract manufacturing of biologics for third parties, and small amounts of equity financing. On February 5, 2014, we successfully completed our initial public offering, placing 5,400,000 shares at \$17 per share, raising total gross proceeds of \$91.8 million (€67.3 million) and net proceeds of \$85.4 million (€62.6 million) after commissions but before expenses. From our first institutional venture capital financing in 2006 until our initial public offering, we funded our operations primarily through private and public placements of equity securities, and other convertible debt securities, in the aggregate amount of €204.1 million (\$269.8 million). During this period, we also received total other income, consisting principally of government grants and subsidies, of €6.5 million, and total nonrefundable collaboration funding of €17.0 million. Our predecessor entity, Amsterdam Molecular Therapeutics (AMT) N.V., or AMT, completed an initial public offering of its ordinary shares on Euronext Amsterdam in 2007 and subsequently delisted from that exchange in 2012. We acquired the business of AMT in the first half of 2012.

As of September 30, 2014, we had cash and cash equivalents of €62.8 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.

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We had a net loss of  $\pounds$ 25.9 million in the first nine months of 2014 and  $\pounds$ 20.0 million for the same period of 2013. As of September 30, 2014, we had an accumulated deficit of  $\pounds$ 169.9 million. We anticipate that our expenses will increase substantially in the future as we:

- · Conduct a Phase I/II clinical trial of AMT-060 for hemophilia B in collaboration with Chiesi;
- pursue the pre-clinical and clinical development of product candidates for cardiovascular diseases targeting S100A1, a novel target we obtained as part of our acquisition of InoCard, which took place on July 31, 2014;
- Pursue the pre-clinical and clinical development of product candidates for cardiovascular diseases targeting S100A1, a novel target we obtained as part of our acquisition of InoCard, which took place on July 31, 2014
- · complete our EMA-mandated post-approval clinical trial of Glybera and maintain an LPLD patient registry;
- conduct a clinical trial of Glybera, either as part of the EMA-mandated post-approval clinical trial or separately, to obtain data needed to file a BLA for Glybera with the FDA;
- seek marketing approval for Glybera in the United States and other countries;
- advance the preclinical and clinical development of our other product candidates, most of which are at relatively early stages of development, and seek to discover and develop additional product candidates;
- · seek marketing approval for any product candidates that successfully complete clinical trials;
- exercise our options to acquire rights and pursue development of certain product candidates, the development of which is currently being conducted and funded by third parties;
- acquire or in-license rights to new therapeutic targets or product candidates;
- enter into collaboration agreements with third parties to collaborate on the research and development of potential product candidates;
- exercise our options to acquire rights and pursue development of certain product candidates, the development of which is currently being conducted and funded by third parties;
- · acquire or in-license rights to new therapeutic targets or product candidates;
- establish a sales, marketing and medical affairs infrastructure in the United States;
- fund the ongoing operations of our new manufacturing facility in Lexington, Massachusetts;
- · fund expenses in connection with our collaboration with 4D Molecular Therapeutics;
- maintain, expand and protect our intellectual property portfolio, including in-licensing additional intellectual property rights from third parties;
- hire additional personnel, particularly in our manufacturing, research, clinical development, medical affairs, commercial and quality control groups; and
- · add operational, financial and management information systems and related finance and compliance personnel.

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#### **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  $\leq 17.0$  million in upfront payments from Chiesi under these agreements, as well as a  $\leq 14.0$  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  $\leq 2.0$  million upfront payment in recognition of our past expenditures incurred in developing the product. In addition, we are eligible to earn up to  $\leq 42.0$  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 €5.7 million as of September 30, 2014, until the earlier of repayment in full of such amount and 2019.

#### Hemophilia B agreement

Under the Hemophilia B agreement, we granted to Chiesi an exclusive license, for the European Union and specified countries other than the United States, to co-develop and exclusively commercialize AMT-060, a gene therapy product for the treatment of hemophilia B. We received a  $\leq 15.0$  million upfront payment under this agreement. Of this amount,  $\leq 5.0$  million related to the future development of our hemophilia B product candidate and  $\leq 10.0$  million related to the use of our manufacturing capacity for our hemophilia B product candidate. In addition, we will share equally with Chiesi specified development expenses attributable to the hemophilia B program according to a defined development plan and budget, including expenses associated with preclinical and clinical studies as well as development and regulatory milestone payments associated with existing in-license agreements. We will receive payments from Chiesi for commercial quantities of our hemophilia B product candidate we manufacture and supply to them, if we receive regulatory approval for such product candidate. We estimate that the amount we would retain, net of cost of goods sold, including third party royalties and related amounts, will be between 25% and 35% of the revenues from sales of such product by Chiesi, varying by country of sale. We and Chiesi have agreed to negotiate a separate supply and distribution agreement in respect of the potential commercialization of our hemophilia B product candidate prior to

dosing the first patient in any pivotal study. We are not entitled to any milestone payments under this co-development agreement.

#### Strategic Collaboration: 4D Molecular Therapeutics

In January 2014, we entered into a collaboration and license agreement with 4D Molecular Therapeutics, or 4D, for the discovery and optimization of nextgeneration AAV vectors. Under this agreement, we have an exclusive license to 4D's existing and certain future know-how and other intellectual property for the delivery of AAV vectors to CNS or liver cells for the diagnosis, treatment, palliation or prevention of all diseases or medical conditions. Under this collaboration, the 4D team, including Dr. David Schaffer, 4D's co-founder and Professor of Chemical and Biomolecular Engineering at the University of California, Berkeley, has established a laboratory, which we are funding at a cost of approximately \$3.0 million in aggregate through 2016, to identify next generation AAV vectors. We are also required to make payments for pre-clinical, clinical and regulatory milestones under the collaboration as well as to pay single-digit royalties. In addition, we have granted options to purchase an aggregate of 609,744 ordinary shares in connection with this collaboration, and will recognize resulting share-based payment expense through 2016. To the extent that the collaboration is successful, we may also incur additional third party costs in developing any product candidates and also in preparing, filing and prosecuting additional patent applications.

#### **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.

#### InoCard Acquisition

In July 2014 we acquired InoCard GmbH, an early-stage biotechnology company focused on the development of gene therapy approaches for cardiac disease. InoCard was founded in

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December 2013 as a spin-off of the University of Heidelberg. InoCard has developed a novel gene therapy through preclinical proof of concept, for the onetime treatment of congestive heart failure (CHF). InoCard founders Prof. Patrick Most und Prof. Hugo Katus have joined uniQure as Managing Director of uniQure in Germany and Chairman of the Scientific Advisory Board, for Cardiovascular Diseases, respectively.

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

#### **Financial Operations Overview**

#### Revenues

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

During the nine months ended September 30, 2014, we recognized collaboration revenues of  $\pounds$ 2.5 million associated with development activities that were reimbursable by Chiesi under our co-development agreement for hemophilia B. We expect to continue to recognize such collaboration revenues going forward, in accordance with our contractual agreements.

During the nine months ended September 30, 2014, we also recognized license revenues of  $\notin 0.7$  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  $\notin 15.9$  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 revenue recognition of the related amounts, as we defer the recognition of some upfront payments, including the upfront payments under our Chiesi agreements, and recognize these as revenue when earned or over a defined period, while we treat other revenue, such as milestone payments or service fees, as earned when received. We expect our revenues to vary from quarter to quarter and year to year, depending upon, among other things, the commercial success of Glybera, our success in obtaining marketing approval for Glybera in the United States and additional countries, the structure and timing of milestone events, the number of milestones achieved, the level of revenues earned for ongoing development efforts, any new collaboration arrangements we may enter into and the terms we are able to negotiate with our collaborators. We currently intend to sell Glybera in the United States, if approved, ourselves, in which case we would recognize revenues in the full amount of the sales price. In addition, because LPLD is an orphan disease and we expect that the number of patients that will be treated with Glybera is relatively small, and because we currently expect that we will receive a one-time payment for a single patient treatment, we anticipate that revenues from Glybera may vary significantly

from period to period. Further, because we currently anticipate that LPLD patients will require only a single administration with Glybera, we do not expect to earn recurring revenue from treated patients. We therefore believe that period-to-period comparisons should not be relied upon as indicative of our future revenues.

# **Other Income**

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

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

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

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

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

# Cost of Goods Sold

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

We also include in cost of goods sold amounts that we are required to repay to the Dutch government in respect of a technical development loan that we received in the period from 2000 to 2005 to support the early development of Glybera. As of September 30, 2014, the total amount of principal and interest outstanding was  $\xi$ 5.7 million. Under the terms of this contingent commitment, we are required to make repayments based on the timing and amount

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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  $\in 0.8$  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, 2019. Amounts that remain outstanding as of December 31, 2019, if any, would be forgiven. We have not recorded any liability for these amounts. To the extent we generate revenues from the sale of Glybera, we will recognize a liability and a corresponding charge to cost of goods sold in future periods.

Should we obtain marketing approval in the United States for Glybera, we expect that our 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 September 30, 2014, we incurred an aggregate of  $\pounds$ 120.4 million in research and development expenses. In addition, we began to capitalize our development expenses related to Glybera from March 21, 2013. We capitalized  $\pounds$ 2.7 million of such expenses in the first nine months of 2014, which we expect to begin amortizing once sales of Glybera commence, over the period through September 2032. We allocate our direct research and development expenses to our various programs on the basis of actual external expenses incurred in respect of each program and our allocation of time spent by our research and development team on each program. We do not allocate our overhead expenses to specific development programs. Our research and development expenses mainly relate to the following key programs:

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

- AIP provided to our collaborator, Digna Biotech, for its ongoing Phase I clinical trial in this indication.
- *CNS programs.* We have incurred costs related to the development and manufacture of clinical supplies of AMT-110 for the treatment of Sanfilippo B provided to our collaboration partner, Institut Pasteur, for its ongoing Phase I/II clinical trial. We also incur expenses related to the research and preclinical activities related to our other CNS programs.
- *Technology platform development and other research*. We incur significant research and development costs related to our gene delivery and manufacturing technology platform that are applicable across all of our programs, as well as our other research programs, including intellectual property expenses, depreciation expenses and facility costs. These costs are not allocated to specific projects.

The table below sets forth our direct research and development expenses by program for nine month periods ended September 30, 2013 and 2014:

			Nine months ended September 30,		
(€ in thousands, except percentages)	2013 (restated)	%	2014	%	Change %
Glybera program*	1,665	17.4	5,675	23.8	6.4
Hemophilia B program	1,226	12.8	4,042	17.2	4.4
AIP program	219	2.3	116	0.5	(1.8)
CNS programs	804	8.4	311	1.3	(7.1)
Technology platform development and research programs	5,659	59.1	13,596	57.2	(1.9)
Total	9,573	100	23,740	100	

\*Excludes capitalized development expenses.

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

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

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

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

Over the period through 2016, we anticipate that we will incur external expenses related to the further development of Glybera, including implementation of the patient registry, initiation and conduct of the post-approval clinical trial and additional development work to seek FDA approval, of approximately  $\xi$ 7.0 million; in addition, we will incur significant related employee expenses.

In addition, in connection with our collaboration and license agreement with 4D Molecular Therapeutics, we are incurring expenses to fund a joint research effort with 4D. Further, we granted options to purchase an aggregate of 609,744 of our ordinary shares to two consultants who will be providing services to us in connection with that agreement. The fair value of these options will vest over a three-year future service period, and will have a significant impact on our expenses recognized. Finally, to the extent certain pre-clinical, clinical and regulatory milestones are met, we will make milestone payments to 4D.

# Selling, General and Administrative Expenses

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

well as the costs related to the sales and marketing efforts we intend to undertake in the United States in advance of potential marketing approval for Glybera from the FDA.

# Other Gains / Losses-Net

Other gains / losses—net 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. 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 expenses in 2013 consisted primarily of interest due on our convertible notes, losses on the fair value measurements of our derivative instruments, and, to a lesser extent, the interest component of finance leases. In 2014 finance expenses were driven by the interest due on the Hercules venture debt loan.

#### **Results of Operations**

#### Comparison of the nine months ended September 30, 2013 and 2014

	Nine montl ended September 3		
(€ in thousands, except percentages)	as restated 2013	2014	Change %
Revenues:			
License revenues	220	662	201
Collaboration revenues	1,831	2,551	39
Total revenues	2,051	3,213	57
Cost of goods sold	(800)		
Other income	562	598	6
Expenses:			
Research and development expenses	(9,573)	(23,740)	148
Selling, general and administrative expenses	(7,511)	(8,035)	7
Other gains / losses, net	(269)	3,694	
Operating result	(15,540)	(24,270)	56
Finance income	48	125	160
Finance expense	(4,470)	(1,734)	(61)
Net loss	(19,962)	(25,879)	30

#### Revenues

License revenues for the nine months ended September 30, 2014 were  $\notin 0.7$  million, a 201% increase from the  $\notin 0.2$  million for the nine months ended September 30, 2013. The Chiesi license payment was received in July 2013, and therefore the nine-month period ending September 30, 2013 includes only a partial period of amortization, while the 2014 period reflects a full period of amortization.

Collaboration revenues for the nine months ended September 30, 2014 were €2.6 million, a 39% increase from the €1.8 million for the nine months ended September 30, 2013. This increase reflects increased reimbursement from Chiesi of development activities in connection with our Hemophilia B program.

#### **Cost of Goods Sold**

Cost of goods sold of  $\notin 0.8$  million in the nine months ended September 30, 2013 consisted of the recognition of a repayment obligation to the Dutch government with respect to a portion of a technical development loan. This repayment obligation was triggered by our entitlement to receive during the second quarter of 2013 a  $\notin 2.0$  million upfront payment from Chiesi in relation to our Glybera program. We had no cost of goods sold in the first nine months of 2014.

#### **Other Income**

Other income for the nine months ended September 30, 2014 was €0.60 million, a 6% increase from the €0.56 million recognized for the nine months ended September 30, 2013. This change reflects a slight increase in employee-related government grants received.

### **Research and Development Expenses**

Research and development expenses for the nine months ended September 30, 2014 were €23.7 million, a 148% increase from the €9.6 million incurred for the nine months ended September 30, 2013. This increase reflected the expansion of our research and development activities to support the planned commercial launch of Glybera in the European Union as well as the further development of Glybera and our pipeline product candidates. In addition, as part of our strategic and license collaboration with 4D Molecular Therapeutics entered into in January 2014, we incurred increased research and development expenses related to certain share-based payments made to 4D Molecular Therapeutics.

Glybera-related raw materials that cannot be used for commercial purposes are expensed; Glybera-related materials, including raw materials, work-inprogress 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.

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#### Selling, General and Administrative Expenses

Selling, general and administrative expenses for the nine months ended September 30, 2014 were  $\in$ 8.0 million, a 7% increase from the  $\notin$ 7.5 million incurred for the nine months ended September 30, 2013. This increase resulted principally from added expenses in legal fees and a full year of additional insurance premium payments incurred after the IPO.

#### Other losses—Net

Other gains / losses—net for the nine months ended September 30, 2014 were a gain of  $\notin$  3.7 million, compared with a loss of  $\notin$  0.3 million for the nine months ended September 30, 2013. This increase reflects changes in the foreign exchange rate between the euro and the U.S. dollar.

#### Finance Income

Finance income for the nine months ended September 30, 2014 was  $\notin$  0.13 million, a 160% increase from the  $\notin$  0.05 million for the nine months ended September 30, 2013. This reflects the increased interest income associated with our higher average cash balance in 2014 compared to 2013.

#### Finance Expense

Finance expense for the nine months ended September 30, 2014 was  $\leq 1.7$  million, a 61% decrease from the  $\leq 4.5$  million for the nine months ended September 30, 2013. This decrease primarily related to the revaluation of the embedded derivatives related to our convertible loans and the Hercules venture debt loan, which totaled  $\leq 3.7$  million during the nine months ended September 30, 2013. The  $\leq 1.7$  million in 2014 related to the interest on the Hercules venture debt loan.

#### Comparison of the three months ended September 30, 2013 and 2014

	Three mon ended September		
(€ in thousands, except percentages)	as restated 2013	2014	Change %
Revenues:			
License revenues	220	221	0
Collaboration revenues	1,073	780	(27)
Total revenues	1,293	1,001	(23)
Cost of goods sold		_	
Other income	171	208	22
Expenses:			
Research and development expenses	(3,152)	(9,514)	202
Selling, general and administrative expenses	(3,394)	(3,218)	(5)
Other gains / losses, net	(304)	3,630	
Operating result	(5,386)	(7,893)	47
Finance income	4	54	1,250
Finance expense	(1,656)	(1,220)	(26)
Net loss	(7,038)	(9,059)	29

### Revenues

License revenues for the three months ended September 30, 2014 were 0.22 million, in line with the 0.20 million for the three months ended September 30, 2013.

Collaboration revenues for the three months ended September 30, 2014 were  $\notin 0.8$  million, a 27% decrease from the  $\notin 1.1$  million for the three months ended September 30, 2013. This decrease reflects the increased development efforts on the HemB program where the costs are partially offset by our collaboration agreement with Chiesi.

# **Other Income**

Other income for the three months ended September 30, 2014 was  $\leq 0.21$  million, a 22% increase from the  $\leq 0.17$  million recognized for the three months ended September 30, 2013. This change reflects a slight increase in employee-related government grants received.

# **Research and Development Expenses**

Research and development expenses for the three months ended September 30, 2014 were €9.5 million, a 202% increase from the €3.2 million incurred for the three months ended September 30, 2013. This increase reflected the expansion of our research and development activities to support the planned commercial launch of Glybera in the European Union as well as the further development of Glybera and our pipeline product candidates. In addition, as part of our strategic and license collaboration with 4D Molecular Therapeutics entered into in January 2014, we incurred increased research and development expenses related to certain stock-based payments made to 4D Molecular Therapeutics.

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

# Selling, General and Administrative Expenses

Selling, general and administrative expenses for the three months ended September 30, 2014 were  $\leq$ 3.2 million, a 5% decrease from the  $\leq$ 3.4 million incurred for the three months ended September 30, 2013. This decrease resulted principally from the timing of and an overall reduction in audit-related expenses.

# Other losses—Net

Other gains / losses—net for the three months ended September 30, 2014 were a gain of &3.6 million, compared with a loss of &0.3 million for the three months ended September 30, 2013. This increase reflects changes in the foreign exchange rate between the euro and the U.S. dollar.

# **Finance** Income

Finance income for the three months ended September 30, 2014 was  $\notin 0.05$  million, compared with  $\notin 0.004$  million for the three months ended September 30, 2013. This reflects the increased interest income associated with our higher average cash balance in 2014 compared to 2013.

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# Finance Expense

Finance expense for the three months ended September 30, 2014 was  $\leq 1.2$  million, a 26% decrease from the  $\leq 1.7$  million for the three months ended September 30, 2013. This decrease primarily related to the revaluation of the embedded derivatives related to our convertible loans and venture loan, which totaled  $\leq 3.7$  million during the nine months ended September 30, 2013; partially offset by an increase in interest payments following the amended and increased venture debt loan with Hercules Technology Growth Corporation.

# 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 in February 2014, we funded our operations primarily through private and public placements of equity securities, and convertible and other debt securities, in the aggregate amount of €204.1 million (\$269.8 million). During this period, we also received total other income, consisting principally of government grants and subsidies, of €6.5 million, and total nonrefundable collaboration funding of €17.0 million, and \$20.0 million (€14.6 million) in venture debt financing.

We had a net loss of  $\leq 25.9$  million in the first nine months of 2014 and  $\leq 20.0$  million in the first nine months of 2013. As of September 30, 2014, we had an accumulated deficit of  $\leq 169.9$  million.

# Cash flows

Our cash and cash equivalents as of September 30, 2014 were €62.8 million. The table below summarizes our consolidated cash flow data for the unaudited nine-month periods ended September 30, 2013 and 2014:

	Nine months ended September 30,			
(€ in thousands)	as restated 2013	2014		
Cash (used in) / generated by operating activities	1,654	(16,919)		
Cash used in investing activities	(4,159)	(16,862)		
Cash provided by financing activities	33,663	69,186		

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

Net cash used in operating activities was &16.9 million in the nine months ended September 30, 2014, compared with net cash generated by operating activities of &1.7 million in the nine months ended September 30, 2013. The change principally reflected the higher net loss and receipt of lease incentives in respect of our Lexington facility in the 2014 period, and the receipt in 2013 of the upfront payment of &17.0 million under our collaboration agreements with Chiesi.

# Net Cash Used in Investing Activities

Net cash used in investing activities was €16.9 million in the nine months ended September 30, 2014, a 273% increase from €4.2 million in the nine months ended September 30, 2013. The increase primarily reflected the build-out of our manufacturing facility in Lexington, Massachusetts.

# Net Cash Generated from Financing Activities

Net cash generated from financing activities was  $\in 69.2$  million in the nine months ended September 30, 2014, a 106% increase from  $\in 33.7$  million in the nine months ended September 30, 2013. The increase primarily reflected the receipt of  $\in 62.0$  million, after commissions and expenses, in connection with our initial public offering in February 2014.

# **Cash and Funding Sources**

The table below summarizes our sources of financing for the nine months ended September 30, 2013 and 2014.

(€ in thousands)	Equity Capital(1)	Convertible Notes	Other Debt	Total
Nine months ended September 30, 2014	62,118		7,184	69,302
Nine months ended September 30, 2013	14,278	11,999	7,492	33,769
Total	76,396	11,999	14,676	103,071

(1) Excludes shares issued upon conversion of convertible notes

Our sources of financing in the nine months ended September 30, 2014 were:

- the issuance and sale of 5,400,000 ordinary shares at an initial public offering price of \$17.00 per share, with net proceeds of €62.0 million, after commissions and expenses;
- an additional venture loan in the principal amount of \$10.0 million from Hercules Technology Growth Capital;
- · As of September 30, 2014, we had debt of €15.7 million, which consisted solely of amounts outstanding under the Hercules Agreement.

# **Funding Requirements**

We believe our cash and cash equivalents will enable us to fund our operating expenses, including our debt repayment obligations as they become due, and capital expenditure requirements, for the 12 months following September 30, 2014. 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, our ability to obtain research and development funding and achieve milestones under these
  agreements and our ability to enter into other such new arrangements in the future;
- the progress and results of our current and planned clinical trials, including for Glybera and AMT-060 for hemophilia B, as well as those of our collaborators;

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- the scope, progress, results and costs of preclinical development, laboratory testing and clinical trials for S100A1 in congestive heart failure and our additional product candidates;
- the number and development requirements of other product candidates that we pursue;
- the cost, timing and outcome of regulatory review of our product candidates, particularly for approval of Glybera in the United States;
- the cost and timing of future commercialization activities by us or our collaborators, including product manufacturing, marketing, sales and distribution, for Glybera and any of our product candidates for which we receive marketing approval in the future;
- the amount and timing of revenue, if any, we receive from commercial sales of any product candidates for which we receive marketing approval in the future;
- expenses in connection with our collaboration with 4D Molecular Therapeutics;
- the costs and timing of preparing, filing and prosecuting patent applications, maintaining and enforcing our intellectual property rights and defending any intellectual property-related claims;
- the extent to which we acquire or in-license other products or technologies; and
- the costs associated with maintaining quality compliance and optimizing our manufacturing processes, including the operating costs associated with our Lexington, Massachusetts manufacturing facility.

We have no committed sources of additional financing, other than our collaboration agreements with Chiesi. 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.

# **Capital Expenditures**

The following table sets forth our capital expenditures for the nine months ended September 30, 2013 and 2014:

	Nine months Septembe	
(€ in thousands)	as restated 2013	2014
Investments in property, plant and equipment	(536)	(13,365)
Investments in intangible assets	(3,623)	(2,129)
Acquisition of Business	—	(1,463)
Total	(4,159)	(16,957)
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In the third quarter of 2014, we completed the build-out a 53,000 square foot leased manufacturing facility in Lexington, Massachusetts. The total construction costs amount to approximately \$16.8 million ( $\in$  13.2 million), of which the landlord has paid \$7.3 million ( $\in$ 5.8 million) in landlord improvements. In addition, we anticipate the total investment in property, plant and equipment to be approximately \$8.2 million ( $\in$ 6.5 million). As of September 30, 2014, we had capitalized \$20.2 million ( $\in$ 15.9 million) and had contractual commitments of a further \$1.7 million ( $\in$ 1.3 million). In addition, we provided a landlord deposit of \$1.2 million).

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

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

# **Contractual Obligations and Commitments**

The table below sets forth our contractual obligations and commercial commitments as of September 30, 2014 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)	301	—	—	—	301
Debt obligations	1,639	5,790	13,067	—	20,496
Operating lease obligations	1,849	1,891	4,342	7,359	15,441
Finance lease obligations	165	177	—	—	342
Construction commitment US Facility	1,300	_	—		1,300
Total	5,254	7,858	17,409	7,359	37,880

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

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

The table above does not include:

- Payments we may be obligated to make under our license or collaboration agreements, other than fixed periodic maintenance costs. Such additional payment obligations may be material.
- 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.

# Hercules Loan and Security Agreements

We are party to a Loan and Security Agreement entered into with Hercules on June 13, 2013. Under the Loan and Security Agreement, we borrowed \$10.0 million ( $\notin$ 7.4 million) from Hercules, bearing interest at a variable rate of the greater of 11.85% or an amount equal to 11.85% plus the prime rate of interest minus 3.25%.

On June 26, 2014, we entered into an amended and restated Loan and Security agreement (which amends and replaces the original Loan Agreement) which increased the aggregate amount that we may borrow up to \$20,000,000 ( $\leq$ 14,600,000), net of expenses for facility charges of 1.00% plus expenses related to legal counsel. The additional amount of \$10,000,000 ( $\leq$ 7,344,000) was received net of expenses of \$218,000 ( $\leq$ 160,000). This resulted in a total cash inflow of \$9,782,000 ( $\leq$ 7,184,000). The new loan commitment is \$20,000,000 with an interest rate of 10.25% and a back-end fee of \$250,000, which matures over a period of 48 months. The interest-only period is 18 months. As the terms of the amended loan agreement changed significantly compared to the original loan agreement (maturity date, interest rate, payback schedule), we fully amortized the unamortized transaction costs at issue, which is required under IAS39, resulting in an extra amortization charge through profit and loss in 2014 of \$193,000 ( $\leq$ 141,000).

The Loan and Security agreement also provides for payment of a maturity charge, the amount of which was reduced in exchange for the issuance to Hercules, on September 24, 2013, of 37,174 warrants, at an exercise price of  $\leq 10.10$  per share. The warrant included in the Loan and Security Agreement is not closely related to the host contract and therefore has been split and accounted-for separately as a financial derivative measured at fair value though profit or loss. The fair value of this derivative as of September 30, 2014 was  $\leq 93,000$  compared to  $\leq 270,000$  on September 30, 2013.

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

The borrowings under the Loan and Security Agreement were classified as non-current borrowings of  $\leq 15.7$  million, net of expenses, as of September 30, 2014. For the nine-month period ended September 30, 2014, we recorded  $\leq 1.1$  million as finance expenses in relation to the Loan and Security Agreement, compared to  $\leq 0.3$  million for the same period in 2013.

The exchange result on the borrowings under the Loan and Security Agreement amounts to €1.1 million.

As of September 30, 2014, as a result of the extension of the interest-only payment period, we no longer classified the borrowings under the Loan and Security Agreement as a current liability.

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

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financing, to make investments in securities or in other companies, to transfer our assets, to perform certain corporate changes, to make loans to employees, officers and directors, and to make dividend payments and other distributions. Further, we are required to keep a minimum cash balance deposited in bank accounts in the United States, equivalent to the lesser of the outstanding balance of principal due and 50% of our worldwide cash reserves. This restriction on the cash reserves only relates to the location of the cash reserves, but all cash reserves are at free disposal of the Company. The Loan and Security Agreement contains default provisions that include the occurrence of a material adverse effect, as defined therein, which would entitle Hercules to declare all principal, interest and other amounts owed by us immediately due and payable. As of September 30, 2014, we were in compliance with these covenants.

# **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  $\leq$ 3.6 million during the grant period. The grant amount bears interest of 5.7% per year and includes a repayment clause in the event we generate revenues from Glybera, during the period from January 1, 2008 through December 31, 2019, based upon a percentage of revenues which are derived from the sale of Glybera, if any. If future amounts received are not sufficient to repay the grant on or prior to December 31, 2019, or if there are no revenues generated from Glybera, the remaining balance will be forgiven. The amount of this contingent commitment as of September 30, 2014 totaled  $\leq$ 5.7 million, comprising the original grant together with accrued interest, less an initial repayment made in the third quarter of 2013. We have not recorded any liability to repay amounts in respect of this contingent commitment. Further amounts may be recognized once revenues related to produce sales at Glybera commence.

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

