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

FORM 10-Q

(Mark One)

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

For the quarterly period ended September 30, 2017

OR

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

For the transition period from _____ to _____

Commission file number: 001-36294

uniQure N.V.
(Exact name of Registrant as specified in its charter)

The Netherlands	**Not applicable**
(State or other jurisdiction of incorporation or organization)	(I.R.S. Employer Identification No.)

Paasheuvelweg 25a,
1105 BP Amsterdam, The Netherlands
(Address of principal executive offices) (Zip Code)

+31-20-240-6000
(Registrant's telephone number, including area code)

Indicate by check mark whether the registrant (1) has filed all reports required to be filed by Section 13 or 15(d) of the Securities Exchange Act of 1934 during the preceding 12 months (or for such shorter period that the registrant was required to file such reports), and (2) has been subject to such filing requirements for the past 90 days. Yes ☒ No ☐.

Indicate by check mark whether the registrant has submitted electronically and posted on its corporate Web site, if any, every Interactive Data File required to be submitted and posted pursuant to Rule 405 of Regulation S-T (§ 232.405 of this chapter) during the preceding 12 months (or for such shorter period that the registrant was required to submit and post such files). Yes ☒ No ☐.

Indicate by check mark whether the registrant is a large accelerated filer, an accelerated filer, a non-accelerated filer, smaller reporting company or an emerging growth company. See the definitions of "large accelerated filer" "accelerated filer" and "smaller reporting company" and "emerging growth company" in Rule 12b-2 of the Exchange Act.

Large accelerated filer ☐	Accelerated filer ☒
Non-accelerated filer (do not check if smaller reporting company)☐	Smaller reporting company ☐
Emerging growth company ☒	

If an emerging growth company, indicate by check mark if the registrant has elected not to use the extended transition period for complying with any new or revised financial accounting standards provided pursuant to Section 13 (a) of the Exchange Act Yes ☒ No ☐

Indicate by check mark whether the registrant is a shell company (as defined in Rule 12b-2 of the Exchange Act.) Yes ☐ No ☒

As of October 30, 2017, the registrant had 30,800,080 shares of ordinary shares, par value €0.05, outstanding.

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SPECIAL CAUTIONARY NOTICE REGARDING FORWARD-LOOKING STATEMENTS

This Quarterly Report on Form 10-Q contains “forward-looking statements” as defined under federal securities laws. Forward-looking statements are based on our current expectations of future event and many of these statements can be identified using terminology such as “believes,” “expects,” “anticipates,” “plans,” “may,” “will,” “projects,” “continues,” “estimates,” “potential,” “opportunity” and similar expressions. These forward-looking statements may be found in Part II, Item 1A “Risk Factors,” Part I, Item 2 “Management’s Discussion and Analysis of Financial Condition and Results of Operations” and other sections of this Quarterly Report on Form 10-Q.

Forward-looking statements involve risks and uncertainties that could cause actual results to differ materially from those projected or implied. The most significant factors known to us that could materially adversely affect our business, operations, industry, financial position or future financial performance include those discussed in Part II, Item 1A “Risk Factors,” as well as those discussed in Part I, Item 2 “Management’s Discussion and Analysis of Financial Condition and Results of Operations” and elsewhere in this report, as well as other factors which may be identified from time to time in our other filings with the Securities and Exchange Commission (“SEC”), including our most recent Annual Report on Form 10-K filed with the Securities and Exchange Commission on March 15, 2017, or in the documents where such forward-looking statements appear. You should carefully consider that information before you make an investment decision.

You should not place undue reliance on these statements, which speak only as of the date that they were made. Our actual results or experience could differ significantly from those anticipated in the forward-looking statements and from historical results, due to the risks and uncertainties described in this Quarterly Report on Form 10-Q for the quarter ended September 30, 2017, and in our Annual Report on Form 10-K for the year ended December 31, 2016, including in “Part I, Item 1A. Risk Factors,” as well as others that we may consider immaterial or do not anticipate at this time. These cautionary statements should be considered in connection with any written or oral forward-looking statements that we may make in the future or may file or furnish with the SEC. We do not undertake any obligation to release publicly any revisions to these forward-looking statements after completion of the filing of this Quarterly Report on Form 10-Q to reflect later events or circumstances or to reflect the occurrence of unanticipated events. All forward-looking statements attributable to us are expressly qualified in their entirety by these cautionary statements.

In addition, with respect to all our forward-looking statements, we claim the protection of the safe harbor for forward-looking statements contained in the Private Securities Litigation Reform Act of 1995.

Part I – FINANCIAL INFORMATION

Item 1. Financial Statements

uniQure N.V.

UNAUDITED CONSOLIDATED BALANCE SHEETS

	September 30, 2017	December 31, 2016
	in thousands, except share and per share amounts	
Current assets		
Cash and cash equivalents	$ 88,934	$ 132,496
Accounts receivable and accrued income	—	3,680
Accounts receivable and accrued income from related party	1,945	5,500
Prepaid expenses	689	996
Other current assets	747	1,274
Total current assets	**92,315**	**143,946**
Non-current assets		
Property, plant and equipment, net	34,653	35,702
Intangible assets, net	9,027	8,324
Goodwill	522	465
Other non-current assets	2,469	1,828
Total non-current assets	**46,671**	**46,319**
Total assets	**$ 138,986**	**$ 190,265**
Current liabilities		
Accounts payable	$ 2,987	$ 5,524
Accrued expenses and other current liabilities	10,165	9,766
Current portion of long-term debt	6,232	605
Current portion of deferred rent	724	684
Current portion of deferred revenue	4,249	6,142
Current portion of contingent consideration	1,017	—
Total current liabilities	**25,374**	**22,721**
Non-current liabilities		
Long-term debt, net of current portion	14,353	19,631
Deferred rent, net of current portion	8,829	6,781
Deferred revenue, net of current portion	67,863	75,612
Contingent consideration, net of current portion	2,593	1,838
Other non-current liabilities	367	51
Total non-current liabilities	**94,005**	**103,913**
Total liabilities	**119,379**	**126,634**
Commitments and contingencies (see note 14)		
Shareholders' equity		
Ordinary shares, €0.05 par value: 60,000,000 shares authorized at September 30, 2017 and December 31, 2016 and 25,635,849 and 25,257,420 shares issued and outstanding at September 30, 2017 and December 31, 2016, respectively.	1,612	1,593
Additional paid-in-capital	471,648	464,653
Accumulated other comprehensive loss	(5,809)	(6,557)
Accumulated deficit	(447,844)	(396,058)
Total shareholders' equity	**19,607**	**63,631**
Total liabilities and shareholders' equity	**$ 138,986**	**$ 190,265**

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

uniQure N.V.

UNAUDITED CONSOLIDATED STATEMENTS OF OPERATIONS AND COMPREHENSIVE LOSS

	Three months ended September 30,		Nine months ended September 30,	
	2017	2016	2017	2016
	in thousands, except share and per share amounts			
License revenues	$ —	$ 246	$ 8	$ 736
License revenues from related party	1,124	993	3,060	2,977
Collaboration revenues	—	1,850	4,638	4,835
Collaboration revenues from related party	1,136	4,132	2,817	7,419
Total revenues	**2,260**	**7,221**	**10,523**	**15,967**
Operating expenses:				
Research and development expenses	(20,103)	(16,604)	(53,963)	(52,531)
Selling, general and administrative expenses	(5,584)	(5,113)	(17,352)	(20,245)
Total operating expenses	**(25,687)**	**(21,717)**	**(71,315)**	**(72,776)**
Other income	14,413	336	14,995	1,256
Other expense	(261)	—	(2,901)	—
Loss from operations	**(9,275)**	**(14,160)**	**(48,698)**	**(55,553)**
Interest income	10	14	33	51
Interest expense	(577)	(507)	(1,583)	(1,685)
Foreign currency gains / (losses), net	(681)	(496)	(1,845)	(1,764)
Other non-operating income, net	—	54	29	701
Loss before income tax expense	**(10,523)**	**(15,095)**	**(52,064)**	**(58,250)**
Income tax benefit / (expense)	278	(177)	278	(401)
Net loss	**$ (10,245)**	$ (15,272)	**$ (51,786)**	**$ (58,651)**
Other comprehensive loss, net of income tax:				
Foreign currency translation adjustments net of tax impact of $0.3 million and $(0.2) million for the three months ended September 30, 2017 and 2016, respectively, and $0.3 million and $(0.4) million for the nine months ended September 30, 2017 and 2016, respectively.	22	2,801	748	3,380
Total comprehensive loss	**$ (10,223)**	**$ (12,471)**	**$ (51,038)**	**$ (55,271)**
Basic and diluted net loss per ordinary share	$ (0.40)	(0.61)	(2.03)	(2.35)
Weighted average shares used in computing basic and diluted net loss per ordinary share	25,632,642	25,142,660	25,546,225	24,972,839

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

uniQure N.V.

UNAUDITED CONSOLIDATED STATEMENTS OF SHAREHOLDERS' EQUITY

	Ordinary shares		Additional paid-in capital	Accumulated other comprehensive income/(loss)	Accumulated deficit	Total shareholders' equity
	No. of shares	Amount				
	in thousands, except share and per share amounts					
Balance at December 31, 2016	**25,257,420**	**$ 1,593**	**$ 464,653**	**$ (6,557)**	**$ (396,058)**	**$ 63,631**
Loss for the period	—	—	—	—	(51,786)	(51,786)
Other comprehensive income	—	—	—	748	—	748
Exercise of share options	294,929	15	1,021	—	—	1,036
Shares distributed during the period	83,500	4	(4)	—	—	—
Share-based compensation expense	—	—	5,978	—	—	5,978
Balance at September 30, 2017	**25,635,849**	**$ 1,612**	**$ 471,648**	**$ (5,809)**	**$ (447,844)**	**$ 19,607**

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

uniQure N.V.

UNAUDITED CONSOLIDATED STATEMENTS OF CASH FLOWS

	Nine months ended September 30,	
	2017	2016
	in thousands	
Cash flows from operating activities		
Net loss	$ (51,786)	$ (58,651)
Adjustments to reconcile net loss to net cash used in operating activities:		
Depreciation, amortization and impairments	6,036	4,543
Share-based compensation expense	5,978	5,020
Change in fair value of derivative financial instruments and contingent consideration	2,704	(2,270)
Unrealized foreign exchange results	1,821	1,779
Change in deferred taxes	-	401
Change in lease incentive	1,938	(468)
Changes in operating assets and liabilities:		
Accounts receivable, prepaid expenses and other current assets	9,477	(4,685)
Inventories	-	406
Accounts payable	(1,440)	32
Accrued expenses and other liabilities	(1,331)	2,029
Deferred revenue	(19,620)	(4,651)
Net cash used in operating activities	(46,223)	(56,515)
Cash flows from investing activities		
Restricted cash	(567)	(617)
Purchase of intangible assets	(1,124)	(1,897)
Purchase of property, plant and equipment	(3,244)	(11,034)
Net cash used in investing activities	(4,935)	(13,548)
Cash flows from financing activities		
Proceeds from issuance of shares	1,036	2,218
Repayment of capital lease obligations	-	(149)
Net cash generated from financing activities	1,036	2,069
Currency effect cash and cash equivalents	6,560	3,919
Net decrease in cash and cash equivalents	(43,562)	(64,075)
Cash and cash equivalents at beginning of period	132,496	221,626
Cash and cash equivalents at the end of period	**$ 88,934**	**$ 157,551**
Supplemental cash flow disclosures:		
Cash paid for interest	**$** 1,130	$ 1,594
Non-cash increases/(decreases) in accounts payables related to purchases of intangible assets and property, plant and equipment	**$** (1,635)	$ 828

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

1 General business information

uniQure (the "Company") was incorporated on January 9, 2012 as a private company with limited liability (besloten vennootschap met beperkte aansprakelijkheid) under the laws of the Netherlands. The Company is a leader in the field of gene therapy and seeks to deliver to patients suffering from rare and other devastating diseases single treatments with potentially curative results. The Company's business was founded in 1998 and was initially operated through its predecessor company, Amsterdam Molecular Therapeutics (AMT) Holding N.V ("AMT"). In 2012, 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. Effective February 10, 2014, in connection with its initial public offering, the Company converted into a public company with limited liability (naamloze vennootschap) and changed its legal name from uniQure B.V. to uniQure N.V.

The Company is registered in the trade register of the Chamber of Commerce (Kamer van Koophandel) in Amsterdam, the Netherlands under number 54385229. The Company's headquarters are in Amsterdam, the Netherlands, and its registered office is located at Paasheuvelweg 25a, Amsterdam 1105 BP, the Netherlands and its telephone number is +31 20 240 6000. The Company's website address is www.uniqure.com.

Effective January 1, 2017, the Company ceased to qualify as a foreign private issuer under the rules and regulations of the Securities Act of 1933, as amended (the "Securities Act"). As a result, as of January 1, 2017, the Company began filing electronically with the Securities and Exchange Commission (the "SEC") its annual reports on Form 10-K, quarterly reports on Form 10-Q, current reports on Form 8-K, and any amendments to those reports filed or furnished pursuant to Section 13(a) or 15(d) of the Securities Exchange Act of 1934, as amended (the "Exchange Act"). Prior to this time, the Company filed its annual report on Form 20-F and furnished quarterly financial reports as an exhibit on Form 6-K with the SEC.

The Company's ordinary shares are listed on the NASDAQ Global Select Market and trades under the symbol "QURE".

2 Summary of significant accounting policies

2.1 Basis of preparation

The Company prepared these unaudited consolidated financial statements in compliance with generally accepted accounting principles in the United States of America ("U.S. GAAP") and applicable rules and regulations of the SEC regarding interim financial reporting. Any reference in these notes to applicable guidance is meant to refer to authoritative U.S. GAAP as found in the Accounting Standards Codification ("ASC") and Accounting Standards Update ("ASU") of the Financial Accounting Standards Board ("FASB").

The unaudited consolidated financial statements are presented in U.S. dollars, except where otherwise indicated. Transactions denominated in currencies other than U.S. dollars are presented in the transaction currency with the U.S. dollar amount included in parenthesis, converted at the foreign exchange rate as of the transaction date.

2.2 Unaudited interim financial information

The accompanying interim financial statements and related disclosures are unaudited, have been prepared on the same basis as the annual financial statements and, in the opinion of management, reflect all adjustments, which include only normal recurring adjustments, necessary for a fair statement of the financial position, results of operations and changes in financial position for the periods presented.

Certain information and footnote disclosures normally included in annual financial statements prepared in accordance with U.S. GAAP have been omitted. The results of operations for the nine months ended September 30, 2017, are not necessarily indicative of the results to be expected for the full year ending December 31, 2017, or for any other future year or interim period. The accompanying financial statements should be read in conjunction with the audited

financial statements and the related notes thereto included in the Company's Annual Report on Form 10-K for the year ended December 31, 2016, filed with the SEC on March 15, 2017.

2.3 Use of estimates

The preparation of the financial statements in conformity with U.S. GAAP requires management to make estimates and assumptions that affect the reported amounts of assets and liabilities, disclosure of contingent assets and liabilities at the date of the financial statements and reported amounts of revenues and expenses during the reporting periods. Actual results could differ from those estimates.

2.4 Accounting policies

The principal accounting policies applied in the preparation of these unaudited consolidated financial statements are described in the Company's audited financial statements as of and for the year ended December 31, 2016, and the notes thereto, which are included in the Company's Annual Report on Form 10-K for the year ended December 31, 2016, filed with the SEC on March 15, 2017. There have been no material changes in the Company's significant accounting policies during the nine months ended September 30, 2017.

2.5 Recent accounting pronouncements

There have been no new accounting pronouncements or changes to accounting pronouncements during the nine months ended September 30, 2017, compared to the recent accounting pronouncements described in Note 2.3.22 of the Company's Annual Report on Form 10-K for the year ended December 31, 2016, which could be expected to materially impact the Company's unaudited condensed consolidated financial statements except the ones discussed below:

In May 2017, the FASB issued ASU 2017-09, Compensation-stock compensation (topic 718)- scope of modification accounting ("ASU 2017-09"), which provides clarity regarding the applicability of modification accounting in relation to share-based payment awards. Under the new guidance, modification accounting is required only if the fair value, the vesting conditions, or the classification of the award (as equity or liability) changes because of the change in terms or conditions. The effective date for the standard is for fiscal years beginning after December 15, 2017, which for the Company is January 1, 2018. Early adoption is permitted. The new standard is to be applied prospectively. The Company does not expect ASU 2017-09 to have a material impact on its consolidated financial statements.

In February 2016, the FASB issued ASU 2016-02, Leases (Topic 842). Under the new guidance, lessees will be required to recognize the assets and liabilities that arise from operating leases. A lessee should recognize in the statement of financial position a liability to make lease payments (the lease liability) and a right-of-use asset representing its right to use the underlying asset for the lease term. For leases with a term of 12 months or less, a lessee is permitted to make an accounting policy election by class of underlying asset not to recognize lease assets and lease liabilities. Lessees and lessors are required to recognize and measure leases at the beginning of the earliest period presented using a modified retrospective approach. ASU 2016-02 will be effective for the Company beginning in the first quarter of 2019 and early application is permitted. The Company does expect ASU 2016-02 to have a material impact on its consolidated financial statements, primarily from recognition of a right-of-use asset and lease liability in the balance sheet and a shift of cash outflows from operating activities to financing activities.

In July 2015, the FASB issued ASU 2015-14, Revenue from Contracts with Customers: Deferral of the Effective Date ("ASU 2015-14"), which deferred the effective date for ASU 2014-09, Revenue from Contracts with Customers ("ASU 2014-09"), by one year. ASU 2014-09 will supersede the revenue recognition requirements in ASC 605, Revenue Recognition, and requires entities to recognize revenue in a way that depicts the transfer of promised goods or services to customers in an amount that reflects the consideration to which the entity expects to be entitled to in exchange for those goods or services. In 2016, the FASB issued ASU 2016-08, 2016-10 and 2016-12, which provided further clarification on ASU 2014-09. ASU 2014-09 is effective for annual reporting periods beginning after December 15, 2017, including interim periods within that reporting period, which for the Company is January 1, 2018.

The Company has one active product development collaboration with Bristol Myers Squibb ("BMS"). ASU 2014-09 provides for two possible implementation methods, (i) full retrospective application to all periods from January 1, 2015

onwards for revenue recognized in relation to collaborations; or (ii) application of the standard from January 1, 2018, onwards to the BMS collaboration with an adjustment to retained earnings as of December 31, 2017, to include the cumulative adjustment to revenue recognized in prior periods in relation to the BMS collaboration.

The Company currently accounts for the BMS collaboration agreement as a revenue arrangement with multiple elements. The Company's substantive deliverables under the BMS collaboration agreement include an exclusive license to its technology in the field of cardiovascular disease, research and development services for specific targets chosen by BMS and general development of the Company's proprietary vector technology, participation in the Joint Steering Committee, and clinical and commercial manufacturing.

The Company is continuing to assess the method of adoption as well as its financial statement disclosures.

3 Fair value measurement

The Company measures certain assets and liabilities at fair value, either upon initial recognition or for subsequent accounting or reporting. U.S. GAAP, requires disclosure of methodologies used in determining the reported fair values, and establishes a hierarchy of inputs used when available. The three levels of the fair value hierarchy are described below:

- Level 1 - Valuations based on unadjusted quoted prices in active markets for identical assets or liabilities that the Company can access at the measurement date.
- Level 2 - Valuations based on quoted prices for similar assets or liabilities in markets that are not active or models for which the inputs are observable, either directly or indirectly.
- Level 3 - Valuations that require inputs that reflect the Company's own assumptions that are both significant to the fair value measurement and are unobservable.

To the extent that valuation is based on models or inputs that are less observable or unobservable in the market, the determination of fair value requires more judgment. Accordingly, the degree of judgment exercised by the Company in determining fair value is greatest for instruments categorized as Level 3. A financial instrument's level within the fair value hierarchy is based on the lowest level of any input that is significant to the fair value measurement.

Items measured at fair value on a recurring basis include financial instruments and contingent consideration. The carrying amount of cash and cash equivalents, accounts receivable from collaborators, prepaid expenses, other assets, accounts payable, accrued expenses and other current liabilities reflected in the consolidated balance sheets approximate their fair values due to their short-term maturities.

The following table sets forth the Company's assets and liabilities that are required to be measured at fair value on a recurring basis as of September 30, 2017, and December 31, 2016:

	Quoted prices in active markets (Level 1)	Significant other observable inputs (Level 2)	Significant unobservable inputs (Level 3)	Total	Classification in consolidated balance sheets
			in thousands		
At December 31, 2016					
Assets:					
Cash and cash equivalents	$ 132,496	$ —	$ —	$ 132,496	
Total assets	132,496	—	—	132,496	
Liabilities:					
Derivative financial instruments - debt	—	—	11	11	Accrued expenses and other current liabilities
Derivative financial instruments - related party	—	—	51	51	Other non-current liabilities
Contingent consideration	—	—	1,838	1,838	
Total liabilities	$ —	$ —	$ 1,900	$ 1,900	
At September 30, 2017					
Assets:					
Cash and cash equivalents	$ 88,934	$ —	$ —	$ 88,934	
Total assets	88,934	—	—	88,934	
Liabilities:					
Derivative financial instruments - debt	—	—	3	3	Accrued expenses and other current liabilities
Derivative financial instruments - related party	—	—	35	35	Other non-current liabilities
Contingent consideration	—	—	3,610	3,610	
Total liabilities	$ —	$ —	$ 3,648	$ 3,648	

Changes in Level 3 items during the nine months ended September 30, 2017, and 2016, are as follows:

	Contingent consideration	Derivative financial instruments	Total
		in thousands	
Balance at December 31, 2016	**$ 1,838**	**$ 62**	**$ 1,900**
(Gains) / losses recognized in profit or loss	2,704	(29)	2,675
Amounts due (presented in Accrued expenses and other current liabilities)	(1,181)	—	(1,181)
Currency translation effects	249	5	254
Balance at September 30, 2017	**$ 3,610**	**$ 38**	**$ 3,648**

	Contingent consideration	Derivative financial instruments	Total
		in thousands	
Balance at December 31, 2015	**$ 2,926**	**$ 837**	**$ 3,763**
(Gains) / losses recognized in profit or loss	(1,569)	(701)	(2,270)
Currency translation effects	87	20	107
Balance at September 30, 2016	**$ 1,444**	**$ 156**	**$ 1,600**

Contingent consideration

In connection with the Company's acquisition of InoCard GmbH ("InoCard") in 2014, the Company recorded contingent consideration related to amounts potentially payable to InoCard's former shareholders. In August 2017, the Company and the former shareholders amended the 2014 sale and purchase agreement to waive certain of the Company's obligations regarding the development of the acquired program pursuant to a plan to be agreed to between the Company and the InoCard former shareholders. The parties also modified the conditions of the agreed milestone payments, including a reduction of the percentage of any future milestone that can be settled in the form of Company ordinary shares from 100% to 50%. The Company recorded $2.3 million in research and development cost in the three and nine months ended September 30, 2017, related to the increase of fair value of the contingent consideration resulting from these modifications.

The amounts payable in accordance with the amended sale and purchase agreement are contingent upon realization of the following milestones:

- Early candidate nomination of product by third party;
- Acceptance of investigational new drug application by the United States Food and Drug Administration or an equivalent filing in defined Western European countries;
- Completion of dosing of all patients in the first clinical study; and
- Full proof of concept of the product in humans after finalization of the first clinical study.

The valuation of the contingent liability is based on significant inputs not observable in the market such as the probability of success ("POS") of achieving certain research milestones (estimated as probable for the first three milestones as of the balance sheet date), the time at which the research milestones are expected to be achieved (ranging from 2018 to 2021), as well as the discount rate applied, which represents a Level 3 measurement. The POS as well as the discount rate both reflect the probability of achieving a milestone as of a specific date. In June 2017, the Company replaced the risk-adjusted discount rate of 30.0% with the Company's weighted average rate of capital of 14.5% to reflect the full integration of the acquired business into the Company's operation. This resulted in a $0.3 million increase of the liability.

Varying the timing of the milestones, the discount rate and the POS of unobservable inputs results in the following fair value changes:

	September 30, 2017
	in thousands
Change in fair value	
Moving out of all milestones by 6 months	$ (231)
Increasing the POS for the first milestone by 20%	1,103
Decreasing the POS for the first milestone by 20%	(1,103)
Reducing the discount rate from 14.5% to 4.5%	1,004
Increasing the discount rate from 14.5% to 24.5%	(590)

	December 31, 2016
	in thousands
Change in fair value	
Moving out of all milestones by 6 months	$ (209)
Increasing the POS for the first milestone by 20%	367
Decreasing the POS for the first milestone by 20%	(367)
Reducing the discount rate from 30% to 20%	638
Increasing the discount rate from 30% to 40%	(309)

Derivative financial instruments

The Company issued derivative financial instruments related to its collaboration with Bristol-Meyers Squibb Company ("BMS") and in relation to the issuance of the Hercules Technology Growth Corp. ("Hercules") loan facility. The fair value of these derivative financial instruments as of September 30, 2017, was $0.0 million (December 31, 2016: $0.1 million), and these derivative financial instruments are described in more detail below.

There were no significant changes in the sensitivity of the fair value from (un)observable inputs as of September 30, 2017, compared to December 31, 2016.

BMS collaboration

On April 6, 2015, the Company entered into several agreements with BMS (the "BMS Agreements"). Pursuant to the terms of the BMS Agreements the Company granted BMS two warrants:

- A warrant allowing BMS to purchase a specific number of uniQure ordinary shares such that its ownership will equal 14.9% immediately after such purchase. The warrant can be exercised on the later of (i) the date on which the Company receives from BMS the Target Designation Fees (as defined in the collaboration agreements) associated with the first six New Targets (as defined in the collaboration agreements); and (ii) the date on which BMS designates the sixth New Target.

- A warrant allowing BMS to purchase a specific number of uniQure ordinary shares such that its ownership will equal 19.9% immediately after such purchase. The warrant can be exercised on the later of (i) the date on which uniQure receives from BMS the Target Designation Fees associated with the first nine New Targets; and (ii) the date on which BMS designates the ninth New Target.

Pursuant to the terms of the BMS Agreements the exercise price, in respect of each warrant, is equal to the greater of (i) the product of (A) $33.84, multiplied by (B) a compounded annual growth rate of 10% and (ii) the product of (A) 1.10 multiplied by (B) the VWAP for the 20 trading days ending on the date that is five trading days prior to the date of a notice of exercise delivered by BMS.

Hercules loan facility

On June 14, 2013, the Company entered into a venture debt loan facility with (the "Original Facility") with Hercules Technology Growth Capital, Inc. ("Hercules") pursuant to a Loan and Security Agreement (the "Loan Agreement") which included a warrant. The warrant was not closely related to the host contract and was accounted for separately as a derivative financial liability measured at fair value though profit or loss. The warrant included in the Original Facility remained in place following the 2014 and 2016 amendments of the loan.

4 Collaboration arrangements and concentration of credit risk

In the three and nine months ended September 30, 2017, the Company generated all collaboration and license revenues from its Collaboration and License Agreement with BMS, and its Co-Development Agreement for hemophilia B with Chiesi Farmaceutici S.p.A. ("Chiesi").

On April 19, 2017, the Company and Chiesi entered into an agreement to terminate the Glybera Commercialization Agreement following the Company's decision to not seek renewal with the European Medicines Agency of the marketing authorization for Glybera by October 2017 ("Glybera Termination Agreement"). In July 2017, the Company and Chiesi terminated their co-development agreement in respect of the hemophilia B program ("hemophilia B Termination Agreement"). As a result, the Company holds the global rights to the development of the hemophilia B program and is not required to provide any further services in relation to the co-development and active contribution to the collaboration by providing technology access in the field of gene therapy to Chiesi.

Since June 2015, BMS has been considered a related party given the significance of its equity investment in the Company.

Services to the Company's collaboration partners are rendered by the Dutch operating entity. Total collaboration and license revenue generated from these partners are as follows:

	Three months ended September 30,		Nine months ended September 30,	
	2017	2016	2017	2016
	in thousands			
Bristol Myers Squibb	$ 2,260	$ 5,125	$ 5,877	$ 10,396
Chiesi Farmaceutici S.p.A	—	2,096	4,646	5,571
Total	**$ 2,260**	**$ 7,221**	**$ 10,523**	$ **15,967**

Amounts owed by these partners in relation to the collaboration are as follows:

	September 30, 2017	December 31, 2016
	in thousands	
Bristol Myers Squibb	$ 1,945	$ 5,500
Chiesi Farmaceutici S.p.A	—	3,680
Total	**$ 1,945**	**$ 9,180**

BMS collaboration

In May 2015, the Company closed a Collaboration and License Agreement with BMS (the "BMS Collaboration Agreement") that provides exclusive access to the Company's gene therapy technology platform for multiple targets in cardiovascular (and other target specific) diseases. The collaboration included the Company's proprietary gene therapy program for congestive heart failure which aims to restore the heart's ability to synthesize S100A1, a calcium sensor and master regulator of heart function, and thereby improve clinical outcomes for patients with reduced ejection fraction. Beyond cardiovascular diseases, the agreement also included the potential for a target exclusive collaboration in other disease areas. In total, the companies may collaborate on ten targets, including S100A1.

The Company is conducting the discovery, non-clinical, analytical and process development activities and is responsible for manufacturing of clinical and commercial supplies using the Company's vector technologies and industrial, proprietary insect-cell based manufacturing platform. BMS reimburses the Company for all its research and development efforts in support of the Collaboration, and will lead the clinical development and regulatory activities across all programs. BMS will also be solely responsible for commercialization of all products from the collaboration.

The Company evaluated the BMS Collaboration Agreement and determined that it is a revenue arrangement with multiple elements. The Company's substantive deliverables under the BMS Collaboration Agreement include an exclusive license to its technology in the field of cardiovascular disease, research and development services for specific targets chosen by BMS and general development of the Company's proprietary vector technology, participation in the Joint Steering Committee, and clinical and commercial manufacturing. The Company concluded that the BMS Collaboration Agreement consists of three units of accounting, including (i) technology (license and target selections), know-how and manufacturing in the field of gene therapy and development and active contribution to the development through the Joint Steering Committee participations, (ii) provision of employees, goods and services for research activities for specific targets and (iii) clinical and commercial manufacturing. The Company determined that the license does not have stand-alone value to BMS without the Company's know-how and manufacturing technology through the participation of the Joint Steering Committee and accordingly, they were combined into one unit of accounting.

License revenue – BMS

As of May 21, 2015, the effective date of the BMS Collaboration Agreement, the Company recorded deferred revenue of $60.1 million. On July 31, 2015, BMS selected the second, third and fourth collaboration targets, triggering a $15.0 million target designation payment to the Company. The Company is entitled to an aggregate of $16.5 million in target designation payments upon the selection of the fifth through tenth collaboration targets. The Company will also be eligible to receive research, development and regulatory milestone payments of up to $254.0 million for S100A1 and up to $217.0 million for each of the other selected targets, if milestones are achieved. The Company determined that the contingent payments under the BMS Collaboration Agreement relating to research, development and regulatory milestones do not constitute substantive milestones and will not be accounted for under the milestone method of revenue recognition. The events leading to these payments solely depend on BMS' performance. Accordingly, any revenue from these contingent payments would be allocated to the first unit of accounting noted above and recognized over the expected performance period.

License revenue is recognized over an expected performance period of 19 years on a straight-line basis commencing on May 21, 2015. The expected performance period is reviewed quarterly and adjusted to account for changes, if any, in the Company´s estimated performance period. The estimated performance period did not change in the nine months ended September 30, 2017.

The Company recognized $1.1 million and $3.1 million of license revenue for the three and nine months ended September 30, 2017, respectively, and compared to $1.0 million and $3.0 million during the same periods in 2016.

Additionally, the Company is eligible to receive net sales-based milestone payments and tiered high single to low double-digit royalties on product sales. These revenues will be recognized when earned.

The royalty term is determined on a licensed-product-by-licensed-product and country-by-country basis and begins on the first commercial sale of a licensed product in a country and ends on the expiration of the last to expire of specified patents or regulatory exclusivity covering such licensed product in such country or, with a customary royalty reduction, ten years after such first commercial sale if there is no such exclusivity.

Collaboration revenue – BMS

The Company provides target-specific research and development services to BMS. Collaboration revenue related to these contracted services is recognized when earned.

The Company generated $1.1 million and $2.8 million of collaboration revenue during the three and nine months ended September 30, 2017, respectively, compared to $4.1 million and $7.4 million during the same periods in 2016.

Manufacturing revenue – BMS

BMS and the Company also entered into a binding term sheet for the Company to supply gene therapy products during the clinical and commercial phase to BMS. Revenues from product sales will be recognized when earned. To date the Company has not supplied any clinical and commercial product to BMS.

Chiesi collaboration

In 2013, the Company entered into two agreements with Chiesi, one for the co-development and commercialization of the hemophilia B program (the “Hemophilia Collaboration Agreement”) and one for the commercialization of Glybera (the “Glybera Agreement”, and together with the Collaboration Agreement, the “Chiesi Agreements”) in Europe and selected territories.

In April 2017, the parties agreed to terminate the Glybera Agreement. Accordingly, the Company will not be required to supply Glybera to Chiesi beyond October 2017. In July 2017, the parties terminated the Hemophilia Collaboration Agreement and the Company reacquired rights associated with its hemophilia B program in Europe and selected territories.

License revenue – Chiesi

Upon the closing of the Chiesi Agreements on September 30, 2013, the Company received €17.0 million ($22.1 million) in non-refundable up-front payments. The Company determined that the up-front payments constituted a single unit of accounting that should be amortized as license revenue on a straight-line basis over the performance period of July 2013 through September 2032. In July 2017, the Company fully released the outstanding deferred revenue and recorded $13.8 million other income during the three and nine months ended September 30, 2017.

The Company recognized $0.0 million and $0.0 million of license revenue during the three and nine months ended September 30, 2017, respectively, compared to $0.2 million and $0.7 million during the same periods in 2016. The Company recognized the license revenue for the nine months ended September 30, 2017, net of a $0.5 million reduction for amounts previously amortized and repaid by the Company in accordance with the Glybera Termination Agreement in 2017.

Collaboration revenue – Chiesi

Prior to the termination of the Hemophilia Collaboration Agreement up to June 30,2017, Chiesi reimbursed the Company for 50% of the agreed research and development efforts related to hemophilia B. These reimbursable amounts have been presented as collaboration revenue.

The Company generated $0.0 million and $4.6 million of collaboration revenue from the co-development of hemophilia B during the three and nine months ended September 30, 2017, respectively, compared to $1.9 million and $4.8 million during the same periods in 2016.

5 Property, plant and equipment

The following table presents the Company's property, plant and equipment as of September 30, 2017, and December 31, 2016:

	September 30, 2017	December 31, 2016
	in thousands	
Leasehold improvements	$ 33,054	$ 30,582
Laboratory equipment	15,952	14,166
Office equipment	2,926	2,710
Construction-in-progress	365	313
Total property, plant, and equipment	52,297	47,771
Less accumulated depreciation	(17,644)	(12,069)
Property, plant and equipment, net	**$ 34,653**	**$ 35,702**

Total depreciation expense was $1.7 million and $5.1 million during the three and nine months ended September 30, 2017, respectively, compared to $1.4 million and $4.1 million during the same periods in 2016. Depreciation expense is allocated to research and development to the extent it relates to the Company's manufacturing facility and equipment. All other depreciation expenses are allocated to selling, general and administrative expense.

6 Intangible assets

The Company's intangible assets include acquired licenses and acquired research and development ("Acquired R&D") and are presented in the following table:

	September 30, 2017	December 31, 2016
	in thousands	
Licenses	$ 9,417	$ 7,799
Acquired research & development	5,512	4,908
Total intangible assets	14,929	12,707
Less accumulated amortization and impairment	(5,902)	(4,383)
Intangible assets, net	**$ 9,027**	**$ 8,324**

Amortization expense was $0.1 million and $0.9 million for the three and nine months ended September 30, 2017, respectively, compared to $0.1 million and $0.4 million during the same periods in 2016. All amortization was included in research and development expenses, except for $0.6 million related to the termination of the Chiesi collaboration, which was presented in other expense in the nine months ended September 30, 2017.

7 Accrued expenses and other current liabilities

Accrued expenses and other current liabilities include the following items:

	September 30, 2017	December 31, 2016
	in thousands	
Accruals for services provided by vendors-not yet billed	$ 3,236	$ 3,824
Personnel related accruals and liabilities	4,481	5,559
Other current liabilities	2,448	383
Total	**$ 10,165**	**$ 9,766**

According to the Glybera Termination Agreement the Company is responsible for terminating the Phase IV post-approval study. The Company accrued $0.9 million (presented as other expenses) during the three months ended June 30, 2017, related to such costs. As of September 30, 2017, the accrual for these amounts was $0.9 million of which $0.6 million is included in other current liabilities.

In addition, as of September 30, 2017 the Company owed $1.2 million to the former shareholders of InoCard (included in other current liabilities on the accompanying balance sheet).

Restructuring plan

In November 2016, the Company announced a plan to restructure its activities resulting from a company-wide strategic review with the aim of refocusing its pipeline, consolidating its manufacturing capabilities into its Lexington, Massachusetts site, reducing operating costs and enhancing overall execution. At various dates between December 2016 and September 2017, the Company entered into termination agreements with certain employees. Depending on the individual fact pattern the Company accrues the related termination costs over the service period or at the date of communication to the employees. Changes in accrued termination benefits (included in research and development expenses) for the nine months ended September 30, 2017, are detailed in the table below.

	Accrued termination benefits
	in thousands
Balance at December 31, 2016	**$ 1,148**
Accrued through profit and loss	1,677
Payments	(1,812)
Currency translation effects	88
Balance at September 30, 2017	**$ 1,101**

8 Long-term debt

On June 14, 2013, the Company entered into a venture debt loan facility with Hercules, which was amended and restated on June 26, 2014, and again on May 6, 2016 (“2016 Amended Facility”). The 2016 Amended Facility extended the maturity date from September 30, 2018, to May 1, 2020. As at September 30, 2017, and December 31, 2016, $20.0 million was outstanding. The interest rate is adjustable and is the greater of (i) 8.25% or (ii) 8.25% plus the prime rate less 5.25%. Under the 2016 Amended Facility, the interest rate will initially be 8.25% per annum with a back-end fee of 4.85% and a facility fee of 0.75% of the outstanding loan amounts. The interest-only payment period expires on November 2017, but can be extended to May 2018 upon the Company raising a cumulative $30.0 million in up-front corporate payments and/or proceeds from equity financings (“Raisings”), and further extended to November 2018 upon the Company raising a cumulative $50.0 million from such Raisings.

The amortized cost of the 2016 Amended Facility, was $20.6 million as of September 30, 2017, compared to $20.2 million as of December 31, 2016, and is recorded net of discount and debt issuance costs. The foreign currency gain on the loan in the three and nine months ended September 30, 2017, was $0.7 million and $2.3 million, respectively, compared to a foreign currency loss of $0.2 million and $0.4 million during the same periods in 2016. The fair value of the loan approximates its carrying amount, as the loan is amortized at a market conforming interest rate and the impact of discounting is insignificant.

Interest expense associated with the 2016 Amended Facility during the three and nine months ended September 30, 2017, was $0.6 million and $1.6 million, respectively, compared to $0.5 million and $1.7 million during the same periods in 2016.

As a covenant in the 2016 Amended Facility, the Company has periodic reporting requirements and is 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 worldwide cash reserves. This restriction on the cash reserves only relates to the location of the cash reserves, and such cash reserves can be used at the discretion of the Company. In combination with other covenants, the 2016 Amended Facility restricts the Company's ability to, among other things, incur future indebtedness and obtain additional debt financing, to make investments in securities or in other companies, to transfer assets, to perform certain corporate changes, to make loans to employees, officers and directors, and to make dividend payments and other distributions. The Company secured the facilities by pledging the shares in its subsidiaries, substantially all its receivables, moveable assets as well as the equipment, fixtures, inventory and cash of uniQure Inc.

9 Shareholders' Equity

On September 15, 2017, the Company filed a prospectus supplement to the prospectus dated May 15, 2017, and entered into a sales agreement (the "Sales Agreement") with Leerink Partners LLC ("Leerink") to establish an "at the market" ("ATM") equity offering program pursuant to which Leerink can sell, with the Company's authorization, up to 5 million ordinary shares at prevailing market prices from time to time. The Company will pay Leerink a commission equal to 3% of the gross proceeds of the sales price of all shares sold through it as sales agent under the Sales Agreement. The Company has not yet sold any ordinary shares under the Sales Agreement and has not received any gross proceeds. The Company capitalized $0.4 million of expenses related to this offering (included in other current assets in the accompanying balance sheet).

10 Share-based compensation

Share-based compensation expense recognized by classification included in the consolidated statements of operations and comprehensive loss was as follows:

	Three months ended September 30,		Nine months ended September 30,	
	2017	2016	2017	2016
	in thousands			
Research and development - employees	$ 1,131	$ 893	$ 2,612	$ 2,670
Selling, general and administrative - employees	1,316	30	3,366	1,680
Research and development - non-employees	—	—	—	670
Total	**$ 2,447**	**$ 923**	**$ 5,978**	**$ 5,020**

Share-based compensation expense recognized by award type was as follows:

	Three months ended September 30,		Nine months ended September 30,	
	2017	2016	2017	2016
Award type	in thousands			
Share options	$ 638	$ 718	$ 2,347	$ 4,408
Restricted share units ("RSUs")	728	88	1,960	323
Performance share units ("PSUs")	1,081	117	1,671	289
Total	**$ 2,447**	**$ 923**	**$ 5,978**	**$ 5,020**

As of September 30, 2017, the unrecognized compensation costs related to unvested awards under the various share-based compensation plans were:

Award type	Unrecognized compensation costs	Weighted-average remaining period for recognition
	in thousands	in years
Share options	$ 7,041	2.52
Restricted share units	4,003	1.64
Performance share units	3,747	1.84
Total	**$ 14,791**	**2.11**

The Company satisfies the exercise of share options and vesting of RSUs and PSUs through newly issued shares.

The Company's share-based compensation plans include the 2014 Amended and Restated Share Option Plan (the "2014 Plan") and inducement grants under Rule 5653(c)(4) of the NASDAQ Global Select Market with characteristics similar to the 2014 Plan (classified as "Other Plans"). The Company previously had a 2012 Equity Incentive Plan (the "2012 Plan") and issued options to purchase ordinary shares to the shareholders of 4D in connection with a collaboration and license agreement between the Company and 4D dated as of January 2014 (classified as "Other Plans").

2014 Plan

Share options

The following table summarizes option activity under the Company's 2014 Plan for the nine months ended September 30, 2017:

	2014 plan	
	Options	Weighted average exercise price
Outstanding at December 31, 2016	**1,812,766**	**$ 12.47**
Granted	949,350	$ 6.17
Forfeited	(348,697)	$ 7.95
Expired	(164,106)	$ 14.46
Exercised	(8,125)	$ 7.49
Outstanding at September 30, 2017	**2,241,188**	**$ 10.38**
Fully vested and exercisable	820,522	$ 12.58
Outstanding and expected to vest	1,420,666	$ 9.10
Total weighted average grant date fair value of options issued during the period (in $ million)		$ 3.5
Grants during the period to directors and officers	641,250	$ 6.43
Proceeds from option sales (in $ million)		—

Options to purchase ordinary shares granted to the Company's non-executive directors, other than those granted upon appointment will vest one year from the date of grant.

The fair value of each option issued was estimated at the date of grant using the Hull & White option pricing model with the following weighted-average assumptions:

	Three months ended September 30,		Nine months ended September 30,	
Assumptions	**2017**	**2016**	**2017**	**2016**
Expected volatility	80%	75%	75% - 80%	75%
Expected terms (in years)	10 years	10 years	10 years	10 years
Risk free interest rate	2.40% - 2.48%	1.52% - 1.89%	2.40% - 2.81%	0.16% - 1.96%
Expected dividends	0%	0%	0%	0%

Restricted Share Units (RSUs)

The following table summarizes the RSUs activity for the nine months ended September 30, 2017:

	RSU	
	Number of shares	**Weighted average grant-date fair value**
Non-vested at December 31, 2016	**307,063**	**$ 9.11**
Granted	428,350	$ 6.00
Vested	(25,000)	$ 18.21
Forfeited	(38,550)	$ 7.15
Non-vested at September 30, 2017	**671,863**	**$ 6.90**
Total weighted average grant date fair value of RSUs issued during the period (in $ million)		$ 2.6
Grants during the period to directors and officers	255,000	$ 5.95

RSUs vest over one to three years. RSUs granted in March 2017 to the Company's Chief Executive Officer will vest equally over two years from the date of grant and RSUs granted to non-executive directors will vest one year from the date of grant. RSUs granted during the period include 255,000 RSUs granted to directors and officers

Performance Share Units (PSUs)

The following table summarizes the PSUs activity for the nine months ended September 30, 2017:

	PSU	
	Number of shares	**Weighted average grant-date fair value**
Non-vested at December 31, 2016	**111,564**	**$ 5.76**
Granted	13,000	$ 4.99
Retired	(12,000)	$ 5.76
Vested	(58,500)	$ 5.76
Non-vested at September 30, 2017	**54,064**	**$ 5.57**
PSUs awarded but not yet earned	437,060	
Total non-vested and discretionary PSUs	491,124	
Total weighted average grant date fair value of PSUs awarded during the period (in $ million)		$ 2.0

In January 2017, the Company awarded PSUs to its executives and other members of senior management. These PSUs are earned based on the Board's assessment of the level of achievement of agreed performance targets through December 31, 2017.

In September 2016, the Company awarded PSUs to its Chief Executive Officer, subject to the successful implementation of the strategic plan. The earning of these PSUs is based on the Board's assessment of the Chief Executive Officer's performance through December 31, 2017.

Other Plans

Under Rule 5653(c)(4) of the NASDAQ Global Select Market, the Company grants share options and RSUs to officers as a material inducement to enter into employment with the Company. In 2017, the Company granted 175,000 inducement RSUs with a grant date fair value of $1.0 million.

The following table summarizes option activity under Other Plans for the nine months ended September 30, 2017:

	Other plans	
	Options	Weighted average exercise price
Outstanding at December 31, 2016	**187,500**	**$ 17.93**
Granted	300,000	$ 6.90
Expired	(62,500)	$ 27.82
Outstanding at September 30, 2017	**425,000**	**$ 8.69**
Fully vested and exercisable	39,062	$ 12.98
Outstanding and expected to vest	385,938	$ 8.25
Total weighted average grant date fair value of options issued during the period (in $ million)		$ 1.2

The fair value of the inducement grant options was estimated at the date of grant using the Hull & White option pricing model with the same assumptions as used in determining the fair value of options issued under the 2014 Plan.

2012 Plan

The following table summarizes option activity under the Company's 2012 Plan for the nine months ended September 30, 2017:

	2012 plan	
	Options	Weighted average exercise price
Outstanding at December 31, 2016	**483,006**	**€ 5.13**
Exercised	(286,804)	€ 3.07
Forfeited	(5,000)	€ 3.07
Expired	(9,000)	€ 3.07
Outstanding, fully vested and exercisable at September 30, 2017	**182,202**	**€ 8.52**

Options exercised under the 2012 plan during the nine months ended September 30, 2017, resulted in total proceeds to the Company of $1.0 million.

11 Income taxes

Deferred tax assets and deferred tax liabilities are recognized based on the expected future tax consequences temporary differences between the financial statement carrying amounts and the income tax basis of assets and liabilities, using current statutory rates. A valuation allowance is recorded against deferred tax assets if it is more likely than not that some or all the deferred tax assets will not be realized. Due to the uncertainty surrounding the realization of the favorable tax attributes in future tax returns, the Company has recorded a full valuation allowance against the Company's otherwise recognizable net deferred tax assets.

12 Basic and diluted earnings per share

Diluted earnings per share is calculated by adjusting the weighted average number of ordinary shares outstanding, assuming conversion of all potentially dilutive ordinary shares. As the Company has incurred a loss, all potentially dilutive ordinary shares would have an antidilutive effect, if converted, and thus have been excluded from the computation of loss per share.

The potentially dilutive ordinary shares are summarized below:

	September 30, 2017	September 30, 2016
	ordinary shares	
BMS warrants	5,286,254	3,442,655
Warrants	37,175	37,175
Stock options under 2012 Plan	182,202	681,057
Stock options under 2014 Plan	2,241,188	2,036,372
Stock options (other)	425,000	325,000
Non-vested and earned RSUs and PSUs	900,927	297,198
Total potential dilutive ordinary shares	**9,072,746**	**6,819,457**

13 Leases

The Company leases various office space and laboratory space under the following operating lease agreements:

Lexington, Massachusetts / United States

In July 2013, uniQure entered into a lease for a facility in Lexington, Massachusetts, United States. The term of the lease commenced in November 2013 and was set for 10 years and is non-cancellable. 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. The lease provides for annual minimum increases in rent, based on a consumer price index.

Amsterdam / The Netherlands

In March 2016, the Company entered into a 16-year lease for a facility in Amsterdam, the Netherlands, and amended this agreement in June 2016. The Company consolidated its three Amsterdam sites into the new site at the end of May 2017. The lease for this facility terminates in 2032, with an option to extend in increments of five year periods. The lease contract provides for annual minimum increases in rent, based on a consumer price index.

As of September 30, 2017, aggregate minimum lease payments for the calendar years and lease incentives received were as follows:

	Lexington	Amsterdam	Total
	in thousands		
2017 (three months remaining)	$ 453	$ —	$ 453
2018	1,849	1,963	3,812
2019	1,903	1,963	3,866
2020	1,956	1,963	3,919
2021 and beyond	6,899	21,595	28,494
Total minimum lease payments	**$ 13,060**	**$ 27,484**	**$ 40,544**
Deferred rent related to lease incentives	$ 5,739	$ 3,814	$ 9,553
Current portion	724	—	*724*

Rent expense is calculated on a straight-line basis over the term of the leases and considers the lease incentives received. Aggregate rent expense was as follows:

	Three months ended September 30,		Nine months ended September 30,	
	2017	2016	2017	2016
	in thousands			
Rent expense-Lexington	$ 276	$ 276	$ 828	$ 828
Rent expense-Amsterdam	555	782	2,080	2,089
Total rent expense	**$ 831**	**$ 1,058**	**$ 2,908**	**$ 2,917**

14 Other commitments

The Company's predecessor entity received a technical development loan from the Dutch government in relation to the development of Glybera. The Company is required to repay the grant through a percentage of revenue derived from product sales of Glybera up to December 31, 2019. Any grant balance remaining at this date will be forgiven. The Company decided not to renew its marketing authorization for Glybera in the European Union, which expires in October 2017. The Company does not expect to derive any revenue from Glybera.

15 Related party transaction

On August 7, 2017, the Company appointed Dr. Sander van Deventer as its Chief Scientific Officer and General Manager of its Amsterdam site. Dr. van Deventer served on the Company's Board until September 14, 2017. Dr. van Deventer currently is Managing Director at the Company's largest shareholder Forbion Capital Partners. Dr. van Deventer has agreed to resign as Managing Partner of Forbion Capital Partners by June 30, 2018, it being understood that he will thereafter continue as a venture partner or similar function with Forbion Capital Partners or its affiliated funds for up to 50% of his time. Dr. van Deventer is entitled to EUR 200,000 gross annual salary ("Base Salary"), including an 8% holiday allowance to be paid annually in May based upon the previous year's gross annual salary. Dr. van Deventer will also be eligible for a bonus amounting to a maximum of 40% of his annual gross salary, such amount to be determined by the Board. On September 20, 2017, Dr. van Deventer was granted an option to purchase 150,000 shares at a price of $8.49, in accordance with the Company's Amended and Restated 2014 Share Incentive Plan.

On October 26, 2017, the Company and our Chief Executive Officer and Executive Director, Matthew Kapusta, entered into an amendment (the "Amendment") to Mr. Kapusta's employment agreement dated December 9, 2014, as previously amended (the "Agreement"). The Amendment changes Mr. Kapusta's severance entitlement in the event of a termination of his employment that occurs within the period that starts ninety days preceding a Change of Control (as defined in the Agreement) and ends one year following a Change of Control. Mr. Kapusta will be entitled in such circumstances to a lump sum payment equal to two times Mr. Kapusta's then-current base salary (as defined in the Agreement) to be paid no later than sixty days after the termination date, his bonus (as defined in the Agreement) for the year of termination pro-rated based upon Mr. Kapusta's termination date, and a lump sum representing and additional two times Mr. Kapusta's bonus , to be paid no later than sixty days following the termination date. Mr. Kapusta's employment agreement previously provided for severance payments of one times his then- current base salary, his pro-rated bonus for the year of termination and a lump-sum payment representing one times his bonus. Mr. Kapusta's other severance entitlements with respect to a termination connected with a Change of Control have not changed.

16 Subsequent event

On October 27, 2017, the Company completed its public offering which was announced on October 23, 2017. The Company issued and sold 5,000,000 ordinary shares at $18.25 per ordinary share, resulting in gross proceeds to the Company of approximately $91.3 million. The net proceeds to the Company from this offering were approximately $85.4 million, after deducting underwriting discounts and commissions and other estimated offering expenses payable by the Company. The Company has granted the underwriters an option to purchase up to 750,000 ordinary shares at the public offering price of $18.25 for within thirty days of the date of the underwriting agreement.

The Company intends to use the net proceeds from this offering to fund the continued clinical development of AMT-061 in hemophilia B and other programs, including AMT-130 in Huntington's disease and other preclinical product candidates focused on rare and orphan diseases and to fund general corporate and working capital purposes.

The above equity financing entitles the Company to extend the interest interest-only payment period of its 2016 Amended Facility by 12 months from November 2017 to November 2018 as described in footnote 8.

Item 2. Management's Discussion and Analysis of Financial Condition and Results of Operations

The following Management's Discussion and Analysis of Financial Condition and Results of Operations ("MD&A") is intended to help the reader understand our results of operations and financial condition. This MD&A is provided as a supplement to, and should be read in conjunction with, our unaudited condensed consolidated financial statements and the accompanying notes thereto and other disclosures included in this Quarterly Report on Form 10-Q, including the disclosures under Part II, Item 1A "Risk Factors", and our audited financial information and the notes thereto included in our Annual Report on Form 10-K for the year ended December 31, 2016, which was filed with the Securities and Exchange Commission (the "SEC"), on March 15, 2017. Our unaudited condensed consolidated financial statements have been prepared in accordance with generally accepted accounting principles in the US ("U.S. GAAP") and unless otherwise indicated are presented in U.S. dollars.

Overview

We are a leader in the field of gene therapy, seeking to develop single treatments with potentially curative results for patients suffering from genetic and other devastating diseases. We are advancing a focused pipeline of innovative gene therapies that have been developed both internally and through partnerships, such as our collaboration with Bristol Myers-Squibb focused on cardiovascular diseases. We have established clinical proof-of-concept in our lead indication, hemophilia B, and achieved preclinical proof-of-concept in Huntington's disease. We believe our validated technology platform and manufacturing capabilities provide us distinct competitive advantages, including the potential to reduce development risk, cost and time to market. We produce our AAV-based gene therapies in our own facilities with a proprietary, commercial-scale, GMP-compliant, manufacturing process. We believe our Lexington, Massachusetts-based facility is one of the world's leading, most versatile, gene therapy manufacturing facilities.

Business Developments

Below is a summary of our recent significant business developments:

Hemophilia B program

On October 19, 2017, we announced that following multi-disciplinary meetings with the U.S. Food and Drug Administration (FDA) and the European Medicines Agency (EMA), we plan to expeditiously advance AMT-061, which combines an AAV5 vector with the FIX-Padua mutant, into a pivotal study in 2018 for patients with severe and moderately severe hemophilia B.

AMT-061 and AMT-060, the latter of which has been tested in 10 patients in an ongoing Phase I/II clinical trial, are identical in structure apart from two nucleotide substitutions in the coding sequence for FIX. The gene variant, referred to as FIX-Padua, expresses a protein with a single amino acid substitution that has been reported in multiple preclinical and nonclinical studies to provide an approximate 8 to 9-fold increase in FIX activity compared to the wild-type FIX protein. All other critical quality attributes of AMT-061 are expected to be comparable to those of AMT-060, as AMT-061 utilizes the same AAV5 capsid and proprietary insect cell-based manufacturing platform.

Based on our meetings with the FDA and EMA we received updates on the clinical and regulatory pathway for AMT-061. The FDA has agreed that AMT-061 will be included under the existing Breakthrough Therapy designation and Investigational New Drug (IND) for AMT-060. The EMA also has agreed that AMT-061 will be included under the current PRIME designation. We achieved general agreement with the FDA and EMA on the proposed pivotal trial plan for AMT-061. The study is expected to be an open-label, single-dose, multi-center, multi-national trial investigating the efficacy and safety of AMT-061 administered to adult patients with severe or moderately severe hemophilia B. The primary objective of the trial is to evaluate AMT-061 for prevention of bleedings. Secondary objectives include additional efficacy and safety aspects. Patients will serve as their own control, with a baseline established during a six-month observational lead-in phase prior to treatment with AMT-061. Concurrent with the start of the six-month lead-in phase of the pivotal study, a short dose-confirmation study is expected to begin in the third quarter of 2018. Three patients will receive a single intravenous (IV) dose of AMT-061 at 2 x 1013 gc/kg and will be evaluated for a period of approximately six weeks to assess FIX activity levels and confirm the dose. Each patient will continue to be followed longer term, and no lead-in phase is required for the dose confirmation study.

AMT-061 nonclinical data demonstrate tolerability and substantial increases in Factor IX (FIX) activity. A Good Laboratory Practices (GLP), nonclinical study of AMT-061 has been performed in non-human primates at four different dose levels up to a dose of 9 x 1013 gc/kg. The purpose of this study was to compare AMT-061 to AMT-060 with respect to liver transduction, circulating FIX protein levels, circulating FIX activity levels and toxicity, after a single intravenous dose with 13- or 26-week observation periods. Data from the study demonstrated a strong correlation between dose and human FIX (hFIX) expression levels, as well as biological activity of the expressed hFIX protein. At equal doses, circulating vector DNA plasma levels, liver distribution, liver cell transduction and hFIX protein expression were comparable for both AMT-060 and AMT-061. Additionally, AMT-061 demonstrated substantial increases in hFIX clotting activity compared to AMT-060, consistent with those previously reported for FIX-Padua. Based on a statistical analysis of the AMT-061 and AMT-060 non-human primate data, as well as the clinical data from the Phase I/II trial of AMT-060, we believe that AMT-061 administered at a dose of 2 x 1013 gc/kg may lead to mean FIX activity of approximately 30 to 50 percent of normal. The study also examined toxicology of AMT-061, including liver enzyme activity, coagulation biomarkers and other safety parameters. Data from the study demonstrated that AMT-061 was well-tolerated with no evidence of any significant toxicological findings. There was no increased thrombin generation or increased fibrin formation or degradation detected during the six months of follow-up. No increase in immunogenicity is expected with AMT-061, as there are no changes in the AAV5 capsid.

We believe that AMT-061 continues to leverage AAV5's favorable tolerability and immunogenicity results. AAV5-based gene therapies have been demonstrated to be generally safe and well-tolerated in a multitude of clinical trials, including three uniQure trials conducted in 22 patients in hemophilia B and other indications. In contrast to data reported using other AAV capsids delivered systemically via IV infusion, no patient treated in clinical trials with our AAV5 gene therapies has experienced any confirmed, T-cell-mediated immune response to the capsid or material loss of FIX activity. An independent clinical trial has demonstrated that AAV5 has the lowest prevalence of preexisting neutralizing antibodies (NAb) compared to other AAV vectors. Data from the Phase I/II study of AMT-060 also demonstrated clinical proof-of-concept in the presence of preexisting NAb to AAV5, suggesting that all, or nearly all hemophilia B patients may be eligible for treatment with AMT-061.

At this time, commercial-scale, GMP manufacturing of AMT-061 clinical material is underway. We have initiated production of multiple clinical-grade batches of AMT-061 in our state-of-the-art Lexington, MA manufacturing facility. Material is being produced at commercial scale and utilizing current Good Manufacturing Practices (cGMP). We expect to begin releasing product for the pivotal trial by the first quarter of 2018. The manufacturing process, controls and methods utilized for AMT-061 are consistent to those previously used for AMT-060. We have achieved alignment with the FDA and EMA on our plan to establish comparability between AMT-061 and AMT-060. We expect to complete our ongoing comparability analysis and plans to submit the data to the agencies for review in the first quarter of 2018. Data reviewed to date support comparability between AMT-061 and AMT-060.

We also announced on October 19, 2017, that we acquired a patent family that broadly covers the FIX-Padua variant and our use in gene therapy for the treatment of coagulopathies, including hemophilia B. We exclusively licensed certain rights, limited to the protein specific claims only, back to the prior owner and retain a non-exclusive right to these claims for gene therapy. This family includes a patent issued in the U.S., as well as pending patent applications in Europe and Canada. We recently filed divisional patent applications that would further strengthen our intellectual property position related to the FIX-Padua variant.

Huntington program (AMT-130)

On April 26, 2017, we presented new preclinical data at the 12th Annual CHDI Huntington's Disease Therapeutics Conference in Malta. Data from the study demonstrate widespread and effective AAV5 vector distribution and extensive silencing of the human mutant huntingtin gene ("HTT") in mini pigs, among the largest Huntington's disease animal models available for testing. The proof-of-concept study was performed by us in collaboration with Prof. Jan Motlik, Director of the Institute of Animal Physiology and Genetics in the Czech Republic and Ralf Reilmann, Founding Director of the George Huntington Institute in Germany.

The study demonstrated that a single administration of AAV5-miHTT resulted in significant reductions in HTT mRNA in all regions of the brain transduced by AMT-130, as well as in the cortex. Consistent with the reduction in HTT mRNA, a clear dose-dependent reduction in mutant huntingtin protein levels in the brain was observed, with similar trends in the cerebral spinal fluid.

In September 2017 AMT-130 received orphan drug designation from the U.S. Food and Drug Administration.

Also in September 2017, we initiated our GLP toxicology study in non-human primates with AMT-130. We expect to complete this study and file an IND with the FDA at the end of 2018.

On October 18, 2017, we presented new preclinical data on AMT-130 at the European Society of Gene and Cell Therapy (ESGCT) 25th Anniversary Congress in Berlin, Germany.

Data from the study demonstrated that following administration of AMT-130 in Huntington's disease mouse models, significant improvements in both motor-coordination and survival were observed, as well as a dose-dependent, sustained reduction in huntingtin protein. AMT-130 comprises an AAV5 vector carrying a DNA cassette encoding an engineered micro RNA (“miHTT”) that silences the human huntingtin protein. The study on functional improvement and sustained huntingtin lowering was performed by members of our research department in collaboration with Charles River Discovery Research Services, Finland.

Intellectual property

In 2017, we acquired intellectual property that broadly covers the Padua FIX variant and its use in gene therapy for the treatment of coagulopathies, including hemophilia B. We exclusively licensed certain rights, limited to the protein specific claims only, back to the prior owner and retain a non-exclusive right to these claims for gene therapy. The intellectual property includes a patent issued in the U.S., as well as pending patent applications in Europe. We recently filed divisional patent applications with the goal of strengthening the intellectual property covering the Padua variant.

In July 2017, we were granted a patent from the United States Patent and Trademark Office. The newly issued Hermens '627 patent significantly expands our leading intellectual property portfolio related to large-scale, highly reproducible manufacturing of AAV in insect cells. This patent, which broadens earlier claims granted in this patent family, is based on research focused on enhancing the genetic stability of the Rep78/52 encoding sequences used to produce AAV vectors in insect cells. The technology covered in the Hermens '627 patent family is currently widely applied in insect cell-based AAV manufacturing.

AAV 5 safety and immunogenicity data

On May 12, 2017, we presented at the American Society of Gene & Cell Therapy’s (“ASGCT”) Annual Meeting in Washington, D.C., new preclinical data demonstrating successful and effective transduction of AAV5 in non-human primates with pre-existing anti-AAV5 neutralizing antibodies (“NABs”). At all observed levels, pre-existing neutralizing antibodies for AAV5 did not have a negative impact on the transduction effectiveness of the AAV5 vector.

This data suggests that patients with pre-existing anti-AAV5 NABs may be able to be successfully treated with AAV5 gene therapies, such as our product candidates in hemophilia B and in Huntington's disease. This development has the potential to significantly expand the applicability of AAV5 gene therapies to nearly all patients, regardless of pre-existing antibodies. In addition, AAV5 also appears to have a more favorable immunogenicity profile, with no immune responses detected across three clinical studies involving intravenous administration to 22 patients. We believe these factors make AAV5 a highly differentiated, best-in-class vector with the potential to more effectively and safely deliver gene therapies to a greater group of patients in need of treatment.

BMS collaboration

We have made continued progress on our research collaboration with Bristol-Myers Squibb (BMS) in congestive heart failure. On August 8, 2017, we announced that preliminary data from a study in large animals demonstrated both DNA delivery and human S100A1 expression in the myocardium after treatments with product produced from our proprietary insect cell, baculovirus manufacturing process. Based on this finding and others, we and BMS intend to advance the product candidate into further preclinical studies, with a goal of initiating a preclinical therapeutic heart failure study as soon as possible.

Chiesi collaboration

On April 20, 2017, we announced that we will not pursue the renewal of the Glybera (“alipogene tiparvovec”) marketing authorization in Europe when it is scheduled to expire on October 25, 2017. We will be responsible for terminating the Phase IV post-approval study. We accrued $0.9 million related to contract termination cost as at June 30, 2017.

On July 26, 2017, we entered into an agreement with Chiesi to reacquire the rights to co-develop and commercialize hemophilia B gene therapy in Europe and other selected territories and to terminate our co-development and license agreement.

Restructuring

Following the completion of our strategic review in November 2016, we announced a strategic restructuring plan aimed at refocusing our pipeline, consolidating our manufacturing operations and enhancing overall execution to drive shareholder value. Between October 31, 2016, and September 30, 2017 we reduced the number of employees with indefinite contracts from 244 to 183. In 2016, we accrued $1.1 million related to termination benefits offered to executive employees. Throughout 2017 we entered into termination agreements with employees, for which we recognized aggregate termination benefits of $1.7 million during 2017. These changes are expected to reduce annual operating expenses by $5.0 to $6.0 million from 2018 onwards.

At the market program

On September 15, 2017, we filed a prospectus supplement to the prospectus dated May 15, 2017, and entered into the Sales Agreement with Leerink to establish an ATM program pursuant to which they are able, with our authorization, to offer and sell up to 5 million ordinary shares at prevailing market prices from time to time. We will pay Leerink a commission equal to 3% of the gross proceeds of the sales price of all shares sold through it as sales agent under the Sales Agreement. We have not yet sold any ordinary shares under the Sales Agreement and has not received any gross proceeds. We capitalized $0.4 million of expenses related to this offering.

Follow-on offering

On October 27, 2017, we completed our public offering which was announced on October 23, 2017. We issued and sold 5,000,000 ordinary shares at $18.25 per ordinary share, resulting in gross proceeds to us of approximately $91.3 million. The net proceeds to us from this offering were approximately $85.4 million, after deducting underwriting discounts and commissions and other estimated offering expenses payable by us. We have granted the underwriters an option to purchase up to 750,000 ordinary shares at the public offering price of $18.25 for within thirty days of the date of the underwriting agreement.

We intend to use the net proceeds from this offering to fund the continued clinical development of AMT-061 in hemophilia B and other programs, including AMT-130 in Huntington’s disease and other preclinical product candidates focused on rare and orphan diseases and to fund general corporate and working capital purposes.

The above equity financing entitles us to extend the interest interest-only payment period of our 2016 Amended Facility by 12 months from November 2017 to November 2018.

Kapusta Employment Agreement Amendment

On October 26, 2017, we and our Chief Executive Officer and Executive Director, Matthew Kapusta, entered into an amendment (the “Amendment”) to Mr. Kapusta’s employment agreement dated December 9, 2014, as previously amended (the “Agreement”). The Amendment changes Mr. Kapusta’s severance entitlement in the event of a termination of his employment that occurs within the period that starts ninety days preceding a Change of Control (as defined in the Agreement) and ends one year following a Change of Control. Mr. Kapusta will be entitled in such circumstances to a lump sum payment equal to two times Mr. Kapusta’s then-current base salary (as defined in the Agreement) to be paid no later than sixty days after the termination date, his bonus (as defined in the Agreement) for the year of termination pro-rated based upon Mr. Kapusta’s termination date, and a lump sum representing and additional two times Mr. Kapusta’s bonus, to be paid no later than sixty days following the termination date. Mr. Kapusta’s employment agreement previously provided for severance payments of one times his then- current base salary, his pro-rated bonus for the year of termination and a lump-sum payment representing one times his bonus. Mr. Kapusta’s other severance entitlements with respect to a termination connected with a Change of Control have not changed.

Financial Overview

Key components of our results of operations include the following:

	Three months ended September 30,		Nine months ended September 30,	
	2017	2016	2017	2016
	in thousands			
Total revenues	$ 2,260	$ 7,221	$ 10,523	$ 15,967
Research and development expenses	(20,103)	(16,604)	(53,963)	(52,531)
Selling, general and administrative expenses	(5,584)	(5,113)	(17,352)	(20,245)
Net loss	(10,245)	(15,272)	(51,786)	(58,651)

As of September 30, 2017, and December 31, 2016, we had cash and cash equivalents of $88.9 million and $132.5 million, respectively. We had a net loss of $10.2 million and $51.8 million during the three and nine months ended September 30, 2017, respectively, compared to $15.3 million and $58.7 million during the same periods in 2016. As of September 30, 2017, and December 31, 2016, we had accumulated deficits of $447.8 million and $396.1 million, respectively. We anticipate that our loss from operations will increase in the future as we:

- Advance AMT-061 into late-stage clinical development. In July 2017, we and Chiesi terminated our Hemophilia Collaboration Agreement. Accordingly, all future development expenses will be borne by us (previously, Chiesi was reimbursing 50% of such costs);
- Complete our IND-enabling studies for our proprietary Huntington's disease gene therapy program and initiate clinical studies;
- Advance multiple research programs related to gene therapy candidates targeting liver-directed, central nervous system ("CNS") and cardiovascular disorders;
- Continue to enhance and optimize our technology platform, including our manufacturing capabilities, next-generation viral vectors and promoters, and other enabling technologies;
- Seek marketing approval for any product candidates that successfully complete clinical trials;
- Acquire or in-license rights to new therapeutic targets or product candidates;
- Maintain, expand and protect our intellectual property portfolio, including in-licensing additional intellectual property rights from third parties; and
- Build-out our clinical, medical and regulatory capabilities in the U.S.

See "Results of Operations" below for a discussion of the detailed components and analysis of the amounts above.

Critical Accounting Policies and Estimates

In preparing our consolidated financial statements in accordance with U.S. GAAP and pursuant to the rules and regulations promulgated by the "SEC" our management makes assumptions, judgments and estimates that can have a significant impact on our net income/loss and affect the reported amounts of certain assets, liabilities, revenue and expenses, and related disclosures. On an ongoing basis, we evaluate our estimates and judgments, including those related to the BMS collaboration agreement, share-based payments, contingent consideration, valuation of derivative financial instruments, and research and development expenses. We base our estimates on historical experience, known trends and events and various other factors that are believed to be reasonable under the circumstances, the results of which form the basis for making judgments about the carrying values of assets and liabilities that are not clear from other sources. Actual results may differ from these estimates under different assumptions or conditions. In making estimates and judgments, management employs critical accounting policies. During the nine months ended September 30, 2017, there were no material changes to our critical accounting policies as reported in our Annual Report on Form 10-K for the year ended December 31, 2016, which was filed with the SEC on March 15, 2017.

Revenues

We recognize collaboration revenues associated with development activities that are reimbursable by Chiesi (up to the July 2017 termination of the collaboration) and BMS under our respective collaboration agreements.

We recognize license revenues associated with the amortization of the non-refundable upfront payment, target designation fees and research and development milestone payments we received or might receive from BMS. The timing of these cash payments may differ from the recognition of revenue, as revenue is deferred and recognized over the duration of the performance period. We recognize other revenue, such as sales milestone payments or service fees, as earned when realizable.

Research and development expenses

We expense research and development costs ("R&D") as incurred. Our R&D expenses generally consist of costs incurred for the development of our target candidates, which include:

- Employee-related expenses, including salaries, benefits, travel and share-based compensation expense;
- Costs incurred for laboratory research, preclinical and nonclinical studies, clinical trials, statistical analysis and report writing, and regulatory compliance costs incurred with clinical research organizations and other third-party vendors;
- Costs incurred to conduct consistency and comparability studies;
- Costs incurred for the start-up and validation of our Lexington facility;
- Costs incurred for the development and improvement of our manufacturing processes and methods;
- Costs associated with our research activities for our next-generation vector and promoter platform;
- Costs incurred, including share-based compensation expense, under our collaboration and license agreement with 4D Molecular Therapeutics;
- Changes in the fair value of the contingent consideration related to our acquisition of InoCard;
- Facilities, depreciation and other expenses, which include direct and allocated expenses for rent and maintenance of facilities, insurance and other supplies; and
- Amortization of intangible assets.

Our research and development expenses primarily consist of costs incurred for the research and development of our product candidates, which include:

- *AMT-060/061 (hemophilia B)*. We initiated a Phase I/II clinical trial of AMT-060 for the treatment of hemophilia B in the first quarter of 2015. In October 2017, we announced our intention to initiate a pivotal study in 2018 with AMT-061, a gene therapy including an AAV5 vector containing the Padua-FIX gene variant. We incurred costs related to the research, development and production of AMT-061. In July 2017, we and Chiesi terminated our Hemophilia Collaboration Agreement. Accordingly, all future development expenses will be borne by us (previously, Chiesi was reimbursing 50% of such costs);
- *AMT-130 (Huntington's disease).* We have incurred costs related to preclinical and nonclinical studies of AMT-130;
- *AMT-126 (congestive heart failure).* In the third quarter of 2014, we started to incur costs related to the preclinical development of product candidates targeting the S100A1 gene. Since May 2015, all costs related to the program are reimbursed by BMS under the collaboration agreement;
- *Preclinical research programs*. We incur costs related to the research of multiple preclinical gene therapy product candidates with the potential to treat certain rare and other serious medical conditions;
- *Technology platform development and other related research*. We incur significant research and development costs related to vector design, manufacturing and other aspects of our modular gene therapy technology platform that are applicable across all our programs; and
- AMT-110 (*Sanfilippo B*). We incurred costs related to the development and manufacture of clinical supplies of AMT-110 for the Phase I/II clinical trial. We suspended this program in late 2016.

Our research and development expenses may vary substantially from period to period based on the timing of our research and development activities, including manufacturing campaigns, regulatory submissions and enrollment of patients in clinical trials. The successful development of our product candidates is highly uncertain. Estimating the nature, timing or cost of the development of any of our product candidates involves considerable judgement 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;
- our ability to successfully manufacture and scale-up production;
- clinical trial protocols, speed of enrollment and resulting data;
- the effectiveness and safety of our product candidates;
- the timing of regulatory approvals; and
- our ability to agree to ongoing development budgets with collaborators who share the costs of our development programs.

A change in the outcome of any of these variables with respect to our product candidates that we may develop could mean a significant change in the expenses and timing associated with the development of such product candidate.

Selling, general and administrative expenses

Our general and administrative expenses consist principally of employee, office, consultancy, legal and other professional and administrative expenses. We incur expenses associated with operating as a public company, including expenses for personnel, legal, accounting and audit fees, board of directors' costs, directors' and officers' liability insurance premiums, NASDAQ listing fees, expenses related to investor relations and fees related to business development and maintaining our patent and license portfolio. We began the commercialization of Glybera in September 2015 and decided to cease commercialization in April 2017. During this period, we incurred selling and marketing costs related to maintaining a patient registry and conducting a post-approval, Phase IV study for Glybera.

Other items, net

Our other income consists of payments to subsidize our research and development efforts as well as income recognized in relation to the termination of our collaboration with Chiesi in 2017.

Our other expense principally consists of expenses incurred in relation to terminating the marketing of our Glybera program in 2017, as well as costs associated with exiting our prior Amsterdam facilities and exiting our Heidelberg site.

Results of Operations

Comparison of the three months ended September 30, 2017, and 2016

The following table presents a comparison of the three months ended September 30, 2017, and 2016.

	Three months ended September 30,		
	2017	2016	2017 vs 2016
	in thousands		
Total revenues	$ 2,260	$ 7,221	$ (4,961)
Operating expenses:			
Research and development expenses	(20,103)	(16,604)	(3,499)
Selling, general and administrative expenses	(5,584)	(5,113)	(471)
Total operating expenses	**(25,687)**	**(21,717)**	**(3,970)**
Other income	14,413	336	14,077
Other expense	(261)	—	(261)
Loss from operations	**(9,275)**	**(14,160)**	**4,885**
Other non-operating items, net	(1,248)	(935)	(313)
Loss before income tax benefit / (expense)	**(10,523)**	**(15,095)**	**4,572**
Income tax benefit / (expense)	278	(177)	455
Net loss	**$ (10,245)**	**$ (15,272)**	**$ 5,027**

Revenue

Our revenue for the three months ended September 30, 2017, and 2016 was as follows:

	Three months ended September 30,		
	2017	2016	2017 vs 2016
	in thousands		
License revenue	$ 1,124	$ 1,239	$ (115)
Collaboration revenue Chiesi	—	1,850	(1,850)
Collaboration revenue BMS	1,136	4,132	(2,996)
Total revenues	**$ 2,260**	**$ 7,221**	**$ (4,961)**

We expect to continue to recognize approximately $1.1 million in license revenue each quarter from upfront payments and target designation fees received from BMS in the second and third quarters of 2015. Following the termination of our collaboration with Chiesi in July 2017, we did not recognize any license revenue during the three months ended September 30, 2017. We recognized $0.2 million during the same period in 2016.

Collaboration revenue generated during the three months ended September 30, 2017 from research activities associated with AMT-126, our BMS-partnered S100A1 heart failure program, was $1.1 million compared to $4.1 million for the same period in 2016. The reduction in the current year period was driven by the production of preclinical material during the same period in 2016.

Following the termination of our collaboration with Chiesi in July 2017, we no longer recognize collaboration revenue from our co-development of hemophilia B with Chiesi. We recognized $1.9 million collaboration revenue during the same period in 2016.

Research and development expenses

Research and development expenses for the three months ended September 30, 2017, were $20.1 million compared to $16.6 million for the same period in 2016.

- We incurred $9.5 million in personnel, share-based compensation expenses and consulting cost in the three months ended September 30, 2017, compared to $10.1 million during the three months ended September 30, 2016. The decrease was a combination of $0.8 million one-off expenses related to termination benefits and cost reductions resulting from our restructuring initiated in November 2016;
- We incurred $4.8 million in external services and cost related to the development of our product candidates in the three months ended September 30, 2017, compared to $4.8 million for the same period in 2016;
- We recorded $2.3 million in expenses related to an increase in the fair value of the contingent consideration owed to the sellers of InoCard business in the three months ended September 30, 2017, compared to a decrease of $1.8 million for the same period in 2016. The increase in 2017 is partially driven by the amendment of the sale and purchase in August 2017; and
- We incurred $3.6 million operating expenses and depreciation expenses related to our rented facilities in the three months ended September 30, 2017, compared to $3.5 million for the same period in 2016. The increase in 2017 is driven primarily by the additional costs associated with the refurbishment of our new Amsterdam facility, which commenced in March 2016.

Selling, general and administrative expenses

Selling, general and administrative expenses for the three months ended September 30, 2017, were $5.6 million compared to $5.1 million for the same period in 2016.

- Our expenses related to employees, contractors and consultants in the three months ended September 30, 2017, were $1.9 million compared to $2.0 million for the same period in 2016;
- We incurred $1.3 million of share-based compensation expenses in the three months ended September 30, 2017, compared to $0.0 million for the same period in 2016. The increase was related to equity instruments offered to employees during the last twelve months as well as a one-time reversal of share-based compensation expenses of $0.7 million in the three months ended September 30, 2016, because of forfeitures of options to acquire 800,000 ordinary shares by our former CEO;
- We incurred $0.9 million of professional fees in the three months ended September 30, 2017, compared to $1.4 million for the same period in 2016. The decrease was primarily related to costs incurred in 2016 associated with our conversion from IFRS to U.S. GAAP; and
- We incurred $0.0 million of costs associated with the Glybera global registry and Phase IV study during the three months ended September 30, 2017, compared to $0.5 million during the same period in 2016. These costs were presented as selling, general and administrative costs prior to May 2017, after which time they are presented as other expense.

Other items, net

We recognized $13.8 million of income in the three months ended September 30, 2017, following the termination of our collaboration with Chiesi in July 2017. The income related to the full amortization of the outstanding deferred revenue. We recognized no such income in the same period in 2016.

We recognized $0.3 million of income from payments received from European authorities to subsidize our research and development efforts in the Netherlands in the three months ended September 30, 2017, compared to $0.3 million for the same period in 2016.

We recognized other net expenses of $0.3 million related to various exit activities in the three months ended September 30, 2017. We did not recognize any such expenses in the same period in 2016.

Other non-operating items, net

We recognize interest income associated with our cash and cash equivalents.

We hold monetary items and enter into transactions in foreign currencies, predominantly in euros and U.S. dollars. We recognize foreign exchange results related to changes in these foreign currencies.

We issued warrants to Hercules in 2013 and to BMS in 2015. We recognize changes in the fair value of these warrants within other non-operating income / (expense).

Our other non-operating items, net, for the three months ended September 30, 2017, and 2016 were as follows:

	Three months ended September 30,		
	2017	2016	2017 vs 2016
	in thousands		
Interest income	$ 10	$ 14	$ (4)
Interest expense Hercules long-term debt	(577)	(507)	(70)
Foreign currency losses	(681)	(496)	(185)
Other non-operating income	—	54	(54)
Total other non-operating income / (expense), net	**$ (1,248)**	$ **(935)**	$ **(313)**

We recognized a net foreign currency loss related to our borrowings from Hercules and our cash and cash equivalents of $0.7 million during the three months ended September 30, 2017, compared to a net loss of $0.5 million during the same period in 2016.

In the three months ended September 30, 2017, we did not recognize any gain or loss related to fair value changes of warrants compared to a gain of $0.1 million for the same period in 2016.

Comparison of the nine months ended September 30, 2017, and 2016

The following table presents a comparison of the nine months ended September 30, 2017, and 2016.

	Nine months ended September 30,		
	2017	2016	2017 vs 2016
	in thousands		
Total revenues	$ 10,523	$ 15,967	$ (5,444)
Operating expenses:			
Research and development expenses	(53,963)	(52,531)	(1,432)
Selling, general and administrative expenses	(17,352)	(20,245)	2,893
Total operating expenses	**(71,315)**	**(72,776)**	**1,461**
Other income	14,995	1,256	13,739
Other expense	(2,901)	—	(2,901)
Loss from operations	**(48,698)**	**(55,553)**	**6,855**
Other non-operating items, net	(3,366)	(2,697)	(669)
Loss before income tax benefit / (expense)	**(52,064)**	**(58,250)**	**6,186**
Income tax benefit / (expense)	278	(401)	679
Net loss	**$ (51,786)**	**$ (58,651)**	**$ 6,865**

Revenue

Our revenue for the nine months ended September 30, 2017, and 2016 was as follows:

	Nine months ended September 30,		
	2017	2016	2017 vs 2016
	in thousands		
License revenue	$ 3,068	$ 3,713	$ (645)
Collaboration revenue Chiesi	4,638	4,835	(197)
Collaboration revenue BMS	2,817	7,419	(4,602)
Total revenues	**$ 10,523**	**$ 15,967**	**$ (5,444)**

In association with the upfront payments and target designation fees received from BMS in the second and third quarters of 2015, we recognized $3.1 million in license revenue during the nine months ended September 30, 2017, compared to $3.0 million during the same period in 2016. Following the termination of our collaboration with Chiesi in July 2017, we no longer recognize license revenue in association with the upfront fees received in 2013. We recognized $0.0 million license revenue during the nine months ended September 30, 2017, compared to $0.7 million during the same period in 2016. We recognized our license revenue for the nine months ended September 30, 2017, net of a $0.5 million reduction for amounts previously amortized in relation to the $2.3 million up-front payments that we were required to repay in accordance with the termination of the Glybera Termination Agreement.

Collaboration revenue generated during the nine months ended September 30, 2017, from research activities associated with AMT-126, our BMS-partnered S100A1 heart failure program, was $2.8 million compared to $7.4 million for the same period in 2016. The reduction in the current year period was driven by the timing of various preclinical activities as well as the production of preclinical material during the same period in 2016.

Research and development expenses

Research and development expenses for the nine months ended September 30, 2017, were $54.0 million compared to $52.5 million for the same period in 2016.

- We incurred $27.9 million in personnel, share-based compensation expenses and consulting cost in the nine months ended September 30, 2017, compared to $29.5 million during the nine months ended September 30, 2016. The decrease was a combination of $1.7 million one-off expenses related to termination benefits and cost reductions resulting from our restructuring initiated in November 2016;
- We incurred $11.9 million in external services and costs related to the development of our product candidates in the nine months ended September 30, 2017, compared to $14.2 million for the same period in 2016. The reduction was primarily driven by lower costs of manufacturing drug substance to supply our studies;
- We incurred $10.8 million operating expenses and depreciation expenses related to our rented facilities in the nine months ended September 30, 2017, compared to $10.3 million for the same period in 2016. The increase was driven primarily by additional costs incurred during the first months of 2017 associated with the refurbishment of our new Amsterdam facility;
- We recorded $2.7 million in expenses related to an increase in the fair value of the contingent consideration owed to the sellers of InoCard business in the nine months ended September 30, 2017, compared to a decrease of $1.6 million for the same period in 2016. The increase in 2017 is partially driven by the amendment of the sale and purchase in August 2017; and
- We incurred no share-based compensation expenses related to our collaboration with 4D Molecular Therapeutics in the nine months ended September 30, 2017, compared to $0.7 million for the same period in 2016.

Selling, general and administrative expenses

Selling, general and administrative expenses for the nine months ended September 30, 2017, were $17.4 million compared to $20.2 million for the same period in 2016.

- Our expenses related to employees, contractors and consultants in the nine months ended September 30, 2017, were $6.1 million compared to $6.6 million for the same period in 2016. The decrease was primarily driven by $0.8 million in one-time costs related to the CEO-transition that took place during the first half of 2016;
- We incurred $3.4 million of share-based compensation expenses in the nine months ended September 30, 2017, compared to $1.7 million for the same period in 2016. The increase was related to equity instruments offered to employees during the last twelve months as well as a one-time reversal of share-based compensation expenses of $0.7 million in the three months ended September 30, 2016, because of forfeitures of options to acquire 800,000 ordinary shares by our former CEO;
- We incurred $3.8 million of professional fees in the nine months ended September 30, 2017, compared to $4.4 million for the same period in 2016. The decrease was primarily due to the cost of our conversion from IFRS to U.S. GAAP which we incurred in 2016;
- We incurred legal and settlement costs of $1.9 million in connection with our arbitration proceeding with Extera during the nine months ended September 30, 2016. No such costs were incurred during the nine months ended September 30, 2017; and
- We incurred $0.3 million of costs associated with the Glybera global registry and Phase IV study during the nine months ended September 30, 2017, compared to $2.3 million during the same period in 2016. These costs were presented as selling, general and administrative costs prior to May 2017, after which time they are presented as other expense.

Other items, net

Following the termination of our collaboration with Chiesi in July 2017, we recognized $13.8 million income in the nine months ended September 30, 2017, related to the full amortization of the outstanding deferred revenue. We recognized no such income in the same period 2016.

We recognized $0.9 million in income during the nine months ended September 30, 2017, from payments received from European authorities to subsidize our research and development efforts in the Netherlands compared to $1.3 million for the same period in 2016.

We recognized other expense in the nine months ended September 30, 2017, of $1.8 million related to contractual commitments in relation to terminating the marketing our Glybera program, as well as our collaborations with Chiesi. We did not recognize any such expenses in the same period in 2016.

We accrued $0.6 million of contract termination costs in the nine months ended September 30, 2017, related to vacated facilities at our Amsterdam site. We did not recognize any such expenses in the same period in 2016.

In addition, we accrued $0.5 million related to various exit activities during the nine months ended September 30, 2017. We did not recognize any such expenses in the same period in 2016.

Other non-operating items, net

We recognize interest income associated with cash and cash equivalents.

We hold monetary items and enter into transactions in foreign currencies, predominantly in euros and U.S. dollars. We recognize foreign exchange results related to changes in these foreign currencies.

We issued warrants to Hercules in 2013 and to BMS in 2015. We recognize changes in the fair value of these warrants within other non-operating income / (expense).

Our other non-operating items, net, for the nine months ended September 30, 2017, and 2016 were as follows:

	Nine months ended September 30,		
	2017	2016	2017 vs 2016
	in thousands		
Interest income	$ 33	$ 51	$ (18)
Interest expense Hercules borrowing	(1,583)	(1,685)	102
Foreign currency losses	(1,845)	(1,764)	(81)
Other non-operating income	29	701	(672)
Total other non-operating income / (expense), net	**$ (3,366)**	**$ (2,697)**	**$ (669)**

We recognized a net foreign currency loss related to our borrowings from Hercules and our cash and cash equivalents of $1.8 million during the nine months ended September 30, 2017, compared to a net loss of $1.8 million during the same period in 2016.

In the nine months ended September 30, 2017, we recognized no gain or loss related to fair value changes of warrants compared to a gain of $0.7 million for the same period in 2016.

Financial Position, Liquidity and Capital Resources

As of September 30, 2017, we had cash and cash equivalents of $88.9 million. We currently expect that our cash and cash equivalents will be sufficient to fund operations into early 2020. The table below summarizes our consolidated cash flow data for the nine months ended September 30, 2017, and 2016.

	Nine months ended September 30,	
	2017	2016
	in thousands	
Cash and cash equivalents at the beginning of the period	$ 132,496	$ 221,626
Net cash used in operating activities	(46,223)	(56,515)
Net cash used in investing activities	(4,935)	(13,548)
Net cash generated from financing activities	1,036	2,069
Foreign exchange impact	6,560	3,919
Cash and cash equivalents at the end of the period	**$ 88,934**	**$ 157,551**

We have incurred losses and cumulative negative cash flows from operations since our business was founded by our predecessor entity AMT Therapeutics (“AMT”) Holding N.V. in 1998. We had a net loss of $10.2 million and $51.8 million during the three and nine months ended September 30, 2017, respectively, compared to a loss of $15.3 million and $58.7 million during the same periods in 2016. As of September 30, 2017, we had an accumulated deficit of $447.8 million.

Sources of liquidity

From our first institutional venture capital financing in 2006 through September 30, 2017, we funded our operations primarily through private and public placements of equity securities and convertible and other debt securities and to a much lesser extend upfront, target designation or similar payments from our collaboration partners as well as collaboration revenues.

We expect to continue to incur losses and to generate negative cash flows. We have no firm sources of additional financing other than our collaboration agreements with BMS. Until such time, if ever, as we can generate substantial cash flows from successfully commercializing our product candidates, we expect to finance our cash needs through a combination of equity offerings, debt financings, collaborations, strategic alliances and marketing, distribution and licensing arrangements.

In September 2017, we established an “at the market” equity offering program pursuant to which we can sell up to 5 million ordinary shares at prevailing market prices from time to time.

On October 27, 2017, we completed our public offering announced on October 23, 2017. We issued and sold 5,000,000 ordinary shares at $18.25 per ordinary share, resulting in gross proceeds of approximately $91.3 million. The net proceeds to us from this offering were approximately $85.4 million, after deducting underwriting discounts and commissions and other estimated offering expenses payable by us. We granted the underwriters an option to purchase up to 750,000 ordinary shares at the public offering price of $18.25.

We intend to use the net proceeds from this offering to fund the continued clinical development of AMT-061 in hemophilia B and other programs, including AMT-130 in Huntington’s disease and other preclinical product candidates focused on rare and orphan diseases and to fund general corporate and working capital purposes.

The above equity financing entitles us to extend the interest interest-only payment period of its 2016 Amended Facility by 12 months from November 2017 to November 2018.

We are subject to covenants under our Loan Agreement with Hercules, and may become subject to covenants under any future indebtedness that could limit our ability to take specific actions, such as incurring additional debt, making capital expenditures or declaring dividends, which could adversely impact our ability to conduct our business. In addition, our pledge of assets as collateral to secure our obligations under the Hercules loan agreement may limit our ability to obtain debt financing. To the extent we need to finance our cash needs through equity offerings or debt financings, such financing may be subject to unfavorable terms including without limitation, the negotiation and execution of definitive documentation, as well as credit and debt market conditions, and we may not be able to obtain such financing on terms acceptable to us or at all. If financing is not available when needed, including through debt or equity financings, or is available only on unfavorable terms, we may be unable to meet our cash needs. If we raise additional funds through collaborations, strategic alliances or marketing, distribution or licensing arrangements with third parties, we may have to relinquish valuable rights to our technologies, future revenue streams or product candidates or grant licenses on terms that may not be favorable to us. If we are unable to raise additional funds through equity or debt financings when needed, we may be required to delay, limit, reduce or terminate our product development or future commercialization efforts or grant rights to develop and market product candidates that we would otherwise prefer to develop and market ourselves, which could have a material adverse effect on our business, financial conditions, results of operations and cash flows.

Net Cash used in operating activities

Net cash used in operating activities was $46.2 million for the nine months ended September 30, 2017, a reduction of $10.3 million compared to the $56.5 million of cash used in the same period in 2016.

The reduction is primarily due to a $6.7 million favorable change in our net working capital during the nine months ended September 30, 2017, compared to a $2.2 million unfavorable change in our net working capital during the same period in 2016. In addition, we collected $1.1 million in lease incentive payments related to our new Amsterdam facility and reduced our operating expenses during the nine months ended September 30, 2017 by $0.3 million compared to the same period in 2016.

Net cash used in investing activities

In the nine months ended September 30, 2017, we used $4.9 million in our investing activities compared to $13.5 million for the same period in 2016.

	Nine months ended September 30,	
	2017	2016
	in thousands	
Build out of Lexington site	$ (638)	$ (1,483)
Build out of Amsterdam sites	(2,606)	(9,551)
Acquisition of licenses and patents	(1,124)	(1,897)
Restricted cash	(567)	(617)
Total investments	**$ (4,935)**	**$ (13,548)**

In the nine months ended September 30, 2017, we invested $2.6 million in our new facility in Amsterdam and $9.6 million in the same period 2016.

Net cash generated from financing activities

During the nine months ended September 30, 2017, we received $1.0 million from the exercise of options to purchase ordinary shares in relation to our share incentive plans compared to $2.2 million in the same period 2016.

Funding requirements

We believe our cash and cash equivalents as of September 30, 2017, will enable us to fund our operating expenses including our debt repayment obligations as they become due and capital expenditure requirements, for at least the next twelve months. Our future capital requirements will depend on many factors, including but not limited to:

- the potential to receive future consideration pursuant to our collaboration with BMS, which is largely contingent on achieving certain research, development, regulatory and sales milestones;
- our ability to enter into collaboration arrangements in the future;
- the scope, timing, results and costs of our current and planned clinical trials, including those for AMT-061 in hemophilia B and AMT-130 in Huntington's disease;
- the scope, timing, results and costs of preclinical development and laboratory testing of our additional product candidates, including our S100A1 gene therapy candidate for the treatment of heart failure;
- the need for any additional tests, studies, or trials beyond those originally anticipated to confirm the safety or efficacy of our product candidates and technologies;
- the cost, timing and outcome of regulatory reviews associated with our product candidates;
- the cost and timing of future commercialization activities, including product manufacturing, marketing, sales and distribution of 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, or our collaboration partner, receives marketing approval in the future;
- the costs and timing of preparing, filing, expanding, acquiring, licensing, maintaining, enforcing and prosecuting patents and patent applications, as well as defending any intellectual property-related claims;
- the repayments of the principal amount of our venture debt loan with Hercules, which will contractually start in December 2018 and will run through May 2020;
- the extent to which we acquire or in-license other businesses, products, product candidates or technologies;
- the costs associated with maintaining quality compliance and optimizing our manufacturing processes, including the operating costs associated with our Lexington, Massachusetts manufacturing facility;
- the costs associated with recent and future hiring of senior management and other personnel,
- the timing, costs, savings and operational implications of the corporate restructuring we are implementing following the completion of our strategic review last year.

Contractual obligations and commitments

The table below sets forth our contractual obligations and commercial commitments as of September 30, 2017, that are expected to have an impact on liquidity and cash flows in future periods.

	Undefined	Less than 1 year	Between 1 and 2 years	Between 2 and 5 years	Over 5 years	Total
			in thousands			
Debt obligations (including $3.6 million interest payments)	$ —	$ 7,888	$ 8,848	$ 6,869	$ —	$ 23,605
Operating lease obligations	—	3,308	3,852	11,877	21,507	40,544
Contingent consideration (nominal amount)	15,949	—	—	—	—	15,949
Total	**$ 15,949**	**$ 11,196**	**$ 12,700**	**$ 18,746**	**$ 21,507**	**$ 80,098**

Due to uncertainty of the timing of achieving certain contractual milestones, the contingent consideration of $15.9 million related to our acquisition of InoCard (later renamed uniQure GmbH) is considered to have an undefined contractual maturity. As of September 30, 2017, we expect the milestone obligations will become payable between 2018 and 2021. When due, 50% of the obligations can be settled either in cash or in a variable number of our shares. As of September 30, 2017, we recorded this obligation at its fair value of $3.6 million. In addition, we recorded $1.2 million owed to the former shareholders of Inocard (presented as other current liabilities).

We also have obligations to make future payments to third parties that become due and payable on the achievement of certain development, regulatory and commercial milestones (such as the start of a clinical trial, filing of a Biologics License Application, approval by the FDA or product launch). We have not included these commitments on our balance sheet or in the table above because the achievement and timing of these milestones is not fixed and determinable.

We enter into contracts in the normal course of business with clinical research organizations (“CROs”) for preclinical research studies and clinical trials, research supplies and other services and products for operating purposes. These contracts generally provide for termination on notice, and therefore are cancelable contracts and not included in the table of contractual obligations and commitments.

The Company’s predecessor entity received a technical development loan from the Dutch government in relation to the development of Glybera. The Company needs to repay the grant through a percentage of revenue derived from product sales of Glybera up to December 31, 2019. Any grant balance remaining at this date will be forgiven. We have decided not to renew our marketing authorization for Glybera in the European Union, which expires in October 2017. We do not expect to derive any revenue from Glybera or to be required to make any repayments under this loan.

Off-Balance Sheet Arrangements

As of September 30, 2017, we did not have any off-balance sheet arrangements as defined in Item 303(a) (4) of Regulation S-K.

Item 3. Quantitative and Qualitative Disclosures about Market Risk

We are exposed to a variety of financial risks in the normal course of our business, including market risk (including currency, price and 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.

Our market risks and exposures to such market risks during the nine months ended September 30, 2017, has not materially changed from our market risks and our exposure to market risk discussed in Part II, Item 7A of our Annual Report on Form 10-K for the year ended December 31, 2016, which was filed with the SEC on March 15, 2017.

Item 4. Controls and Procedures

Evaluation of Disclosure Controls and Procedures

Our management, with the participation of our chief executive and chief finance officer ("CEO"), evaluated the effectiveness of our disclosure controls and procedures (as defined in Rules 13a-15(e) and 15d-15(e) under the Securities Exchange Act of 1934, as amended (the "Exchange Act")) as of September 30, 2017. Based on such evaluation, our CEO has concluded that as of September 30, 2017, our disclosure controls and procedures were effective to ensure that information required to be disclosed by it in reports the Company files or submits under the Exchange Act is recorded, processed, summarized and reported within the time periods specified in the rules and forms of the SEC, and that such material information is accumulated and communicated to the Company's management, including its Principal Executive Officer and Principal Financial Officer, to allow timely decisions regarding required disclosure. Because of the inherent limitations in all control systems, any controls and procedures, no matter how well designed and operated, can provide only reasonable assurance of achieving the desired control objectives, and management necessarily is required to apply its judgment in evaluating the cost-benefit relationship of possible controls and procedures. Furthermore, the Company's controls and procedures can be circumvented by the individual acts of some persons, by collusion of two or more people or by management override of such control, and misstatements due to error or fraud may occur and not be detected on a timely basis.

Changes in Internal Control over Financial Reporting

During the nine months ended September 30, 2017, there were no changes in our internal control over financial reporting (as defined in Rule 13a-15(f) under the Exchange Act) that materially affected, or are reasonably likely to affect, internal control over financial reporting.

Part II – OTHER INFORMATION

Item 1. Legal Proceedings

None.

Item 1A. Risk Factors

An investment in our ordinary shares involves a high degree of risk. You should carefully consider the following information about these risks, together with the other information appearing elsewhere in this Quarterly Report on Form 10-Q, including our financial statements and related notes thereto, and the risk factors discussed in Part I, Item 1A "Risk Factors" in our Annual Report on Form 10-K for the year ended December 13, 2016, field with the SEC on March 15, 2017, before deciding to invest in our ordinary shares. We operate in a dynamic and rapidly changing industry that involves numerous risks and uncertainties. The risks and uncertainties described below are not the only ones we face. Other risks and uncertainties, including those that we do not currently consider material, may impair our business. If any of the risks discussed below actually occur, our business, financial condition, operating results or cash flows could be materially adversely affected. This could cause the value of our securities to decline, and you may lose all or part of your investment.

Those risk factors below denoted with a "*" are newly added or have been materially updated from our Annual Report on 10-K filed with the SEC on March 15, 2017.

Risks Related to the Development of Our Product Candidates

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 and non-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. We cannot guarantee that any preclinical tests or clinical trials will be completed as planned or completed on schedule, if at all. A failure of one or more preclinical tests or 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 CROs and clinical trial sites;
- delays in receiving regulatory authority to conduct the clinical trials or a regulatory authority decision that the clinical trial should not proceed;
- delays in obtaining required Institutional Review Board ("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 meeting applicable timelines, properly documenting case files, including the retention of proper case files, and properly monitoring and auditing clinical sites;
- failure of sites or clinical investigators to perform in accordance with good clinical practices ("GCP") or applicable regulatory guidelines in other countries;
- difficulty or delays in patient recruiting into clinical trials;
- delays or deviations 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, undertaking additional new tests or analyses or submitting new types or amounts of clinical data.

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. Such trials and regulatory review and approval take many years. It is impossible to predict when or if any of our clinical trials will demonstrate that product candidates are effective or safe in humans. If the results of our clinical trials are inconclusive, or fail to meet the level of statistical significance required for approval or if there are safety concerns or adverse events associated with our product candidates, we may:

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

Because of the nature of the gene therapies we are developing, regulators may also require us to demonstrate long-term gene expression or clinical efficacy, which may require additional or longer clinical trials and which may not be able to be demonstrated to the regulatory authorities' standards.

Our ability to recruit patients for our trials is often reliant on third parties, such as the pharmacies at our clinical trial sites. These third parties may not have the adequate infrastructure established to handle gene therapy products or to support certain gene therapy product formulations, or may not agree to recruit patients on our behalf.

In addition, we or our collaborator may not be able to locate and enroll enough eligible patients to participate in these trials as required by the FDA, the European Medicines Agency ("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. Because our programs are focused on the treatment of patients with rare or orphan or ultra-orphan diseases, our ability to enroll eligible patients in these trials may be limited or slower than we anticipate considering the small patient populations involved and the specific age range required for treatment eligibility in some indications. 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.

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 required level of 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 during clinical trials, if these results are not reproducible or if our products show diminishing activity over time, our products may not receive approval from the FDA or EMA. 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 because 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.

Fast track product, breakthrough therapy, priority review, or Regenerative Medicine Advanced Therapy ("RMAT") designation by the FDA, or access to the PRIME scheme by the EMA, for our product candidates may not lead to faster development or regulatory review or approval process, and it does not increase the likelihood that our product candidates will receive marketing approval.

We may seek fast track, a breakthrough therapy designation, RMAT designation, and priority review designation and PRIME scheme access for our product candidates if supported by the results of clinical trials. A fast track product designation is designed to facilitate the clinical development and expedite the review of drugs intended to treat a serious or life-threatening condition which demonstrate the potential to address an unmet medical need. A breakthrough therapy is defined as a drug that is intended, alone or in combination with one or more other drugs, to treat a serious or life-threatening disease or condition, and preliminary clinical evidence indicates that the drug may demonstrate substantial improvement over existing therapies on one or more clinically significant endpoints, such as substantial treatment effects observed early in clinical development. A RMAT designation is designed to accelerate approval for regenerative advanced therapies. Priority review designation is intended to speed the FDA marketing application review timeframe for drugs that treat a serious condition and, if approved, would provide a significant improvement in safety or effectiveness. PRIME is a scheme provided by the EMA to enhance support for the development of medicines that target an unmet medical need.

For drugs and biologics that have been designated as fast track products or breakthrough therapies, or granted access to the PRIME schema, interaction and communication between the regulatory agency and the sponsor of the trial can help to identify the most efficient path for clinical development. Sponsors of drugs with fast track products or breakthrough therapies may also be able to submit marketing applications on a rolling basis, meaning that the FDA may review portions of a marketing application before the sponsor submits the complete application to the FDA, if the sponsor pays the user fee upon submission of the first portion of the marketing application. For products that receive a priority review designation, the FDA's marketing application review goal is shortened to six months, as opposed to ten months under standard review. This review goal is based on the date the FDA accepts the marketing application for review, this application validation period typically adds approximately two months to the timeline for review and decision from the date of submission. RAT designations will accelerate approval but the exact mechanisms have not yet been announced by FDA.

Designation as a fast track product, breakthrough therapy, RMAT, PRIME, or priority review product is within the discretion of the regulatory agency. Accordingly, even if we believe one of our product candidates meets the criteria for designation as a fast track product, breakthrough therapy, RMAT, PRIME, or priority review product, the agency may disagree and instead determine not to make such designation. In any event, the receipt of such a designation for a product candidate may not result in a faster development process, review or approval compared to drugs considered for approval under conventional regulatory procedures and does not assure ultimate marketing approval by the agency. In addition, regarding fast track products and breakthrough therapies, the FDA may later decide that the products no longer meet the conditions for qualification as either a fast track product, RMAT, or a breakthrough therapy or, for priority review products, decide that period for FDA review or approval will not be shortened.

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

An element of our strategy is to use our gene therapy technology platform to expand our product pipeline and to progress these candidates through clinical development ourselves or together with our collaborator. 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 collaborator may focus our efforts and resources on potential programs or product candidates that ultimately prove to be unsuccessful. If we do not continue to successfully develop and commercialize product candidates based upon our technology, we may face difficulty in obtaining product revenues in future periods, which could result in significant harm to our financial position and adversely affect our share price.

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

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

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

As of September 30, 2017, a total of three patients reported serious adverse events related to the treatment of AMT-060 in our Phase I/II hemophilia B trial, including one patient with a short, self-limiting fever in the first 24 hours after treatment and two patients with mild, asymptomatic elevations in liver transaminases.

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.

Risks Related to Our Manufacturing

***Our manufacturing facility is subject to significant government regulations and approvals. If we fail to comply with these regulations or maintain these approvals our business will be materially harmed. **

Our manufacturing 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 (“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.

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

The insect-cell based manufacturing process we use to produce our products and product candidates is highly complex and in the normal course is subject to variation or production difficulties. Issues with the manufacturing process, even minor deviations from the normal process, could result in insufficient yield, product deficiencies or manufacturing failures that result in lot failures, insufficient inventory, product recalls and product liability claims.

Many factors common to the manufacturing of most biologics and drugs could also cause production interruptions, including raw materials shortages, raw material failures, growth media failures, equipment malfunctions, facility contamination, labor problems, natural disasters, disruption in utility services, terrorist activities, or acts of god beyond our control. We also may encounter problems in hiring and retaining the experienced specialized personnel needed to operate our manufacturing process, particularly as we transition manufacturing to Lexington, which could result in delays in our production or difficulties in maintaining compliance with applicable regulatory requirements.

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

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

Risks Related to Regulatory Approval of Our Products

We are implementing changes in our lead product candidate for hemophilia B, which may require additional pre-clinical, non-clinical, or clinical studies, or additional chemistry, manufacturing and control development *

We have recently changed our lead product candidate for hemophilia B from AMT-060 (an AAV-5 based vector encoding the wild-type factor IX gene) to a hemophilia B product candidate designated AMT-061 (an AAV5 based vector encoding the FIX-Padua mutant). Both are identical in structure apart from two nucleotide substitutions in the coding sequence for FIX. We believe the FIX-Padua mutant to result in enhanced FIX activity. We have conducted a GLP non-human primate pre-clinical study using AMT-061, which demonstrated a substantial increase in FIX activity over AMT-060. The results of our pre-clinical study using AMT-061 may not be predictive of any future clinical trial results for AMT-061. Our pivotal trial, which will be conducted with AMT-061 may not ultimately provide the desired efficacy results or may reveal adverse events or other safety concerns.

Because of changing our product candidate, we will be required to conduct a clinical study confirming the dosing with AMT-061 and the resulting fold increase in FIX activity. If we are unable to confirm the dose, we might be required to modify the design or extend the study, resulting in a delay of the treatment phase of our pivotal trial.

We have conducted our pre-clinical studies with both AMT-060 and AMT-061 as well our Phase I/II clinical study with AMT-060 with drug product manufactured at our Amsterdam facility. We intend to manufacture AMT-061 for our future clinical studies at our Lexington facility using a scaled-up and modified process. We will need to demonstrate comparability between AMT-061, manufactured at our Lexington facility and AMS-060, manufactured at our Amsterdam, facility to support regulatory approval to commence our Phase III clinical trial.

It is also possible that the applicable regulatory authorities may ultimately not agree with the design or conduct of our comparability, clinical, pre-clinical or non-clinical studies, or with our chemistry, manufacturing, and control development work. The applicable regulatory authorities may also find that the outcome of the foregoing does not support the submission or approval of a marketing application. We are planning to have additional interactions with FDA and the EMA, during which we may receive additional comments, guidance, and recommendations. The approach required by the applicable regulatory authorities, may, however, change in the future due to a variety of reasons, including changes in regulatory policy, the outcome of our studies and continuing development, and how our studies and continuing development efforts are ultimately conducted.

Any of the above could delay the submission of a marketing application, or regulatory authorities may not approve or may require material restrictions on any approvals that are received. Any of the foregoing would materially harm our commercial prospects.

We cannot predict when or if we will obtain marketing approval to commercialize a 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 FDA and other regulatory agencies in the United States, the EMA and other regulatory agencies of the member states of the European Union, and similar regulatory authorities in other jurisdictions. Failure to obtain marketing approval for a product candidate in a specific jurisdiction will prevent us from commercializing the product candidate in that jurisdiction.

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 of any of our product candidates in the United States or other countries, the commercial prospects of our other product candidates may be harmed and our ability to generate revenues will be materially impaired.

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

We believe that all our current product candidates will be viewed as gene therapy products by the applicable regulatory authorities. Gene therapies are relatively new treatments for which regulators do not have extensive experience or standard review and approval processes. The FDA unlike the EMA, does not have an exceptional circumstances approval pathway.

Both the FDA and EMA have demonstrated caution in their regulation of gene therapy treatments, and ethical and legal concerns about gene therapy and genetic testing may result in additional regulations or restrictions on the development and commercialization of our product candidates that are difficult to predict. The FDA and the EMA have issued various guidance documents pertaining to gene therapy products, with which we likely must comply to gain regulatory approval of any of our product candidates in the United States or European Union, respectively. The close regulatory scrutiny of gene therapy products may result in delays and increased costs, and may ultimately lead to the failure to obtain approval for any gene therapy product.

Regulatory requirements affecting gene therapy have changed frequently and may continue to change, and 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. For example, the European Commission conducted a public consultation in early 2013 on the application of EU legislation that governs advanced therapy medicinal products, including gene therapy products, which could result in changes in the data we need to submit to the EMA for our product candidates to gain regulatory approval or change the requirements for tracking, handling and distribution of the products which may be associated with increased costs. In addition, divergent scientific opinions among the various bodies involved

in the review process may result in delays, require additional resources and ultimately result in rejection. These regulatory agencies, committees and advisory groups and the new regulations and guidelines they promulgate may lengthen the regulatory review process, require us to perform additional studies, increase our development costs, lead to changes in regulatory positions and interpretations, delay or prevent approval and commercialization of our product candidates or lead to significant post-approval limitations or restrictions. Delay or failure to obtain, or unexpected costs in obtaining, the regulatory approval necessary to bring a potential product to market could decrease our ability to generate sufficient product revenues to maintain our business.

Our failure to obtain or maintain orphan product exclusivity for any of our product candidates for which we seek this status could limit our commercial opportunity, and if our competitors are able to obtain orphan product exclusivity before we do, we may not be able to obtain approval for our competing products for a significant period.

Regulatory authorities in some jurisdictions, including the European Union and the United States, may designate drugs for relatively small patient populations as orphan drugs. Generally, if a product with an orphan drug designation subsequently receives the first marketing approval for the relevant indication, the product is entitled to a period of market exclusivity, which precludes the EMA or FDA from approving another marketing application for the same drug for the same indication for that period. The EMA, however, may subsequently approve a similar drug for the same indication during the first product's market exclusivity if the EMA concludes that the later drug is clinically superior in that it is shown to be safer, more effective or makes a major contribution to patient care.

Orphan drug exclusivity may be lost if the EMA or FDA determines that the request for designation was materially defective, or if the manufacturer is unable to assure sufficient quantity of the drug to meet the needs of patients with the rare disease or condition or if the incidence and prevalence of patients who are eligible to receive the drug in these markets materially increase.

As appropriate, we intend to seek all available periods of regulatory exclusivity for our product candidates. However, there is no guarantee that we will be granted these periods of regulatory exclusivity or that we will be able to maintain these periods of exclusivity.

The FDA grants product sponsors certain periods of regulatory exclusivity, during which the agency may not approve, and in certain instances, may not accept, certain marketing applications for competing drugs. For example, biologic product sponsors may be eligible for twelve years of exclusivity from the date of approval, seven years of exclusivity for drugs that are designated to be orphan drugs, and/or a six-month period of exclusivity added to any existing exclusivity period or patent life for the submission of FDA requested pediatric data. While we intend to apply for all periods of market exclusivity that we may be eligible for, there is no guarantee that we will receive all such periods of market exclusivity. Additionally, under certain circumstances, the FDA may revoke the period of market exclusivity. Thus, there is no guarantee that we will be able to maintain a period of market exclusivity, even if granted. In the case of orphan designation, other benefits, such as tax credits and exemption from user fees may be available. If we are not able to obtain or maintain orphan drug designation or any period of market exclusivity to which we may be entitled, we will be materially harmed, as we will potentially be subject to greater market competition and may lose the benefits associated with programs.

Risks Related to Our Commercialization

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

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

- successful completion of preclinical studies and clinical trials;
- receipt and maintenance of marketing approvals from applicable regulatory authorities;
- our ability to timely manufacture sufficient quantities according to required quality specifications;
- obtaining and maintaining patent and trade secret protection and non-patent, orphan drug exclusivity for our product candidates;
- obtaining and maintaining regulatory approval for our manufacturing facility in Lexington, Massachusetts;
- launch and commercialization of our products, if approved, whether alone or in collaboration with others;
- identifying and engaging effective distributors or resellers on acceptable terms in jurisdictions where we plan to utilize third parties for the marketing and sales of our product candidates;
- acceptance of our products, if approved, by patients, the medical community and third party payers;
- effectively competing with existing therapies and gene therapies based on safety and efficacy profile;
- achieve value based pricing levels based on durability of expression, safety and efficacy;
- obtaining and maintaining healthcare coverage and adequate reimbursement; and
- complying with any applicable post-approval requirements and maintaining a continued acceptable overall safety profile.

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

***The affected populations for our gene therapies may be smaller than we or third parties currently project, which may affect the size of our addressable markets. **

Our projections of the number of people who have the diseases we are seeking to treat, as well as the subset of people with these diseases who have the potential to benefit from treatment with our therapies, are estimates based on our knowledge and understanding of these diseases. The total addressable market opportunities for these therapies will ultimately depend upon many factors, including the diagnosis and treatment criteria included in the final label, if approved for sale in specified indications, acceptance by the medical community, patient consent, patient access and product pricing and reimbursement. For example, after obtaining marketing authorization for Glybera from the EMA in 2013, various national European authorities denied reimbursement under national insurance schemes.

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

The addressable market for AAV-based gene therapies are also impacted by the prevalence of neutralizing antibodies to the capsids, which are an integral component of our gene therapy constructs. Patients that have pre-existing antibodies to a particular capsid are generally not eligible for administration of a gene therapy that includes this particular capsid. For example, our AMT-061 gene therapy candidate for hemophilia B patients incorporates an AAV5 capsid. In our Phase I/II clinical study of AMT-060, we screened patients for preexisting anti-AAV5 antibodies to determine their eligibility for the trial. Three of the ten patients screened for the study tested positive for anti-AAV5 antibodies on reanalysis, and none of the three tested positive for certain ill-effects from the AAV-based gene therapy, implying that patients who have neutralizing antibodies may be eligible for AAV5-mediated gene transfer. However, we only have been able to test a limited sample of patients and have limited clinical and pre-clinical data, and it is possible that future clinical studies may not confirm these results. This may limit the addressable market for AMT-061 and any future revenues derived from the sale of the product.

Any approved gene therapy we seek to offer may fail to achieve the degree of market acceptance by physicians, patients, third party payers 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 any of our product candidates that receive marketing approval in the future will depend on many factors, including:

- the efficacy and potential advantages of our therapies compared with alternative treatments;
- our ability to convince payers of the long-term cost-effectiveness of our therapies and, consequently, the availability of third party coverage and adequate reimbursement;
- the limitations on use and label requirements imposed by regulators;
- the convenience and ease of administration of our gene therapies compared with alternative treatments;
- the willingness of the target patient population to try new therapies, especially a gene therapy, and of physicians to administer these therapies;
- the strength of marketing and distribution support;
- the prevalence and severity of any side effects;
- limited access to site of service that can perform the product preparation and administer the infusion; and
- any restrictions on the use of our products.

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 our 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 numerous companies focused on developing gene therapies in various indications, including AGTC, Abeona Therapeutics, Adverum Biotechnologies, Asklepios BioPharmaceutical, Audentes Therapeutics, AveXis, Bayer, BioMarin, Bioveratiy, bluebird bio, Dimension Therapeutics, Errant Gene Therapeutics, Expression Therapeutics, Freeline Therapeutics, Genethon, Genzyme, GlaxoSmithKline, Homology Medicines, Lysogene, Megenics, Milo Therapeutics, Nightstarx, Pfizer, REGENXBIO, Renova Therapeutics, Retrosense Therapeutics, Sangamo BioSciences, Shire, Solid Biosciences, Spark Therapeutics, Takara, and Voyager, as well as several companies addressing other methods for modifying genes and regulating gene expression. We may also face competition with respect to the treatment of some of the diseases that we are seeking to target with our gene therapies from protein pharmaceuticals under development at pharmaceutical and biotechnology companies such as Amgen, Bayer, Biogen, BioMarin, Genzyme, Novartis, Novo Nordisk, Pfizer, Shire, and numerous other pharmaceutical and biotechnology firms.

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 FDA, EMA 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

If our collaboration with BMS is not successful or if BMS designates or develops fewer targets than permitted under our collaboration agreement, our development plans, financial position and opportunities for growth may be adversely affected. *

To earn all milestone payments and royalties potentially due under our collaboration with BMS, we are dependent on BMS electing to designate and actively pursue target indications covered by the collaboration and our achievement of all development, clinical and regulatory milestones under the collaboration. If BMS designates or actively pursues fewer development targets, utilizes contract research organizations, instead of our organization, to conduct non-clinical and pre-clinical studies, or if we fail to achieve a significant number of the applicable milestones, the total payments we receive under this collaboration may be materially lower than are potentially payable.

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, or enter into new collaborations, our business could be adversely affected.

We have entered into, and expect in the future to enter into, collaborations with other companies and academic research institutions with respect to important elements of our commercial and development programs. For example, we have a collaboration agreement with BMS for the development and commercialization of gene therapies for cardiovascular and potentially other diseases.

Our existing collaboration, and any future collaborations we enter, may pose several risks, including the following:

- collaborators have significant discretion in determining the efforts and resources that they will apply to these collaborations;
- we generally have limited or no control over the design or conduct of clinical trials sponsored by our current collaborators;

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

If our collaborations do not result in the successful development and commercialization of products or if one of our collaborators terminates its agreement with us, we may not receive future research funding or milestone or royalty payments under the collaboration, and we may lose access to important technologies and capabilities of the collaboration. All the risks relating to product development, regulatory approval and commercialization described herein also apply to the activities of our development collaborators.

Risks Related to Our Intellectual Property

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

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

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

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

The agreements under which we license intellectual property or technology from third parties are complex, and certain provisions in such agreements may be susceptible to multiple interpretations. The resolution of any contract interpretation disagreement that may arise could narrow what we believe to be the scope of our rights to the relevant intellectual property or technology, or increase what we believe to be our financial or other obligations under the relevant agreement, either of which could have a material adverse effect on our business and financial condition.

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

If we are unable to obtain and maintain patent protection for our technology and products, or if the scope of the patent protection is not sufficiently broad, our ability to successfully commercialize our products may be impaired.

We rely upon a combination of in-licensed and owned patents, trade secret protection and confidentiality agreements to protect our intellectual property. Our success depends in a large part on our ability to obtain and maintain this protection in the European Union, the United States and other countries, in part by filing patent applications related to our novel technologies and product candidates. Our patents may not provide us with any meaningful commercial protection, prevent competitors from competing with us or otherwise provide us with any competitive advantage. Our competitors may be able to circumvent our owned or licensed patents by developing similar or alternative technologies or products in a non-infringing manner. Successful challenges to our patents may result in loss of exclusivity or freedom to operate or in patent claims being narrowed, invalidated or held unenforceable, in whole or in part, which could limit our ability to stop others from using or commercializing similar or identical technology and products, or limit the duration of the patent protection of our technology and products.

The patent prosecution process is expensive, time-consuming and uncertain, and we may not be able to file and prosecute all necessary or desirable patent applications at a reasonable cost or in a timely manner. It is also possible that we will fail to identify patentable aspects of our research and development output before it is too late to obtain patent protection. Additionally, given the amount of time required for the development, testing and regulatory review of new product candidates, patents protecting such candidates might expire before or shortly after such candidates are commercialized. As a result, our owned and licensed patent portfolio may not provide us with sufficient rights to exclude others from commercializing products similar or identical to ours.

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

We may become involved in lawsuits to protect or enforce our patents or other intellectual property or third parties may assert their intellectual property rights against us, which could be expensive, time consuming and unsuccessful.

Competitors may infringe our owned or licensed patents or other intellectual property. To counter infringement or unauthorized use, we may be required to file infringement claims, which can be expensive and time consuming. An adverse result in any litigation proceeding could put one or more of our patents at risk of being invalidated, maintained in more narrowly amended form or interpreted narrowly.

Even if resolved in our favor, litigation or other legal proceedings relating to intellectual property claims may cause us to incur significant expenses, increase our operating losses, reduce available resources and could distract our technical and management personnel from their normal responsibilities. In addition, there could be public announcements of the results of hearings, motions or other interim proceedings or developments, which could have an adverse effect on the price of our ordinary shares.

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

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

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

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

In addition, these agreements typically restrict the ability of our collaborators, advisors and consultants to publish data potentially relating to our trade secrets. Our academic collaborators typically have rights to publish data, if 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 product candidates for which we may receive marketing approval.

We anticipate that the cost of treatment using our 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 our product candidates without reimbursement from third party payers, 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 payers, 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 payers have attempted to control costs by limiting coverage and the amount of reimbursement for particular medications and procedures. Increasingly, third party payers 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, particularly considering the new U.S. presidential administration, 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 usually 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. Because of these restrictions, any product candidates for which we may obtain marketing approval may be subject to price regulations that delay or prohibit our or our partners' commercial launch of the product in a particular jurisdiction. In addition, we or our collaborators may elect to reduce the price of our products to increase the likelihood of obtaining reimbursement approvals. If 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 payers, our ability to market and sell our products would be adversely affected and our business would be harmed.

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

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

We also anticipate that 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 payers 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 it is possible that our product candidates will need to be administered only once, there may be situations in which re-administration is required, which may further complicate the pricing and reimbursement for these treatments. In addition, considering the anticipated cost of these therapies, governments and other payers may be particularly restrictive in making coverage decisions. These factors could limit our commercial success and harm our business.

Risks Related to Our Financial Position and Need for Additional Capital

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

We had a net loss of $51.8 million in the nine months ended September 30, 2017, $73.4 million in full year 2016 and $82.1 million in 2015. As of September 30, 2017, we had an accumulated deficit of $447.8 million. To date, we have financed our operations primarily through the sale of equity securities and convertible debt, venture loans, through upfront payments from our collaboration partners and, to a lesser extent, subsidies and grants from governmental agencies and fees for services. We have devoted substantially all our financial resources and efforts to research and development, including preclinical studies and clinical trials. A significant portion of potential consideration under our agreement with BMS is contingent on achieving research, development, regulatory and sales milestones. We expect to continue to incur significant expenses and losses over the next several years, and our net losses may fluctuate significantly from quarter to quarter and year to year.

We anticipate that our expenses will increase substantially as we:

- prepare for a pivotal study for AMT-061, our gene therapy candidate in hemophilia B;
- advance the preclinical development and initiate a clinical study for AMT-130, our product candidate in Huntington's disease;
- progress research programs of additional product candidates targeting liver-directed, CNS and cardiovascular disorders;
- conduct any additional trials or tests beyond those originally anticipated to confirm the safety or efficacy of our product candidates;
- seek marketing approval for any product candidates that successfully complete clinical trials;
- acquire or in-license rights to new therapeutic targets or product candidates;
- build clinical, medical, regulatory affairs, development and commercial infrastructure in the United States;
- maintain, expand and protect our intellectual property portfolio, including in-licensing in license additional intellectual property rights from third parties; and
- incur cost to terminate or retain employees related to restructuring our operations.

We and our collaborator may never succeed in these activities and, even if we do, may never generate revenues that are sufficient to achieve or sustain profitability. Our failure to become and remain profitable would depress the value of our company and could impair our ability to expand our business, maintain our research and development efforts, diversify our product offerings or even continue our operations.

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

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

Adequate capital may not be available to us when needed or may not be available on acceptable terms. Our ability to obtain debt financing may be limited by covenants we have made under our Loan and Security Agreement with Hercules Technology Growth Capital, Inc. ("Hercules") and our pledge to Hercules of substantially all our assets as collateral. If we raise additional capital through the sale of equity or convertible debt securities, our shareholders' ownership interest will be diluted, and the terms of these securities may include liquidation or other preferences that adversely affect the rights of holders of our ordinary shares.

If we raise additional funds through collaborations, strategic alliances, or marketing, distribution or licensing arrangements with third parties, we may have to issue additional equity, relinquish valuable rights to our technologies, future revenue streams, products or product candidates, or grant licenses on terms that may not be favorable to us. If we are unable to raise capital when needed or on attractive terms, we could be forced to delay, reduce or eliminate our research and development programs or any future commercialization efforts, which would have a negative impact on our financial condition, results of operations and cash flows. If our collaboration with BMS is not successful, our development plans, financial position and opportunities for growth may be adversely affected.

The issuance of additional sales of our ordinary shares, or the perception that such issuances may occur, including through our "at the market" offering, could cause the market price of our ordinary shares to fall.

We have entered into the Sales Agreement with Leerink for the offer and sale of up to 5 million ordinary shares from time to time through Leerink, as our sales agent, pursuant to a prospectus supplement to the base prospectus dated May 15, 2017. Leerink is not required to sell any specific number or dollar amount of our ordinary shares, but will use its reasonable efforts, as our agent and subject to the terms of the Sales Agreement, to sell that number of shares upon our request. Sales of the ordinary shares, if any, may be made by any means permitted by law and deemed to be an "at the market" offering as defined in Rule 415 of the Securities Act, and will generally be made by means of brokers' transactions on the NASDAQ Global Select Market or otherwise at market prices prevailing at the time of sale, or as otherwise agreed with Leerink.

We may terminate the Sales Agreement at any time. For the three months ended September 30, 2017, we did not sell any ordinary shares under our "at the market" ("ATM") program. Whether we choose to terminate the Sales Agreement or affect future sales under our ATM program will depend upon a variety of factors, including, among others, market conditions and the trading price of our ordinary shares relative to other sources of capital. The issuance from time to time of these new ordinary shares through our ATM program or in any other equity offering, or the perception that such sales may occur, could have the effect of depressing the market price of our ordinary shares.

Our issuance of ordinary shares under our ATM program may be dilutive, and there may be future dilution of our ordinary shares.

After giving effect to the issuance of ordinary shares under our ATM offering program and the receipt of the expected net proceeds and the use of those proceeds, there may be a dilutive effect on our estimated earnings per share and funds from operations per share in years during which an offering is ongoing. The actual amount of potential dilution cannot be determined at this time and will be based on numerous factors. The market price of our ordinary shares could decline because of issuances of a large number of shares of our ordinary shares after this offering or the perception that such issuances could occur.

Our management will have broad discretion with respect to the use of the proceeds resulting from the issuance of ordinary shares under our ATM program.

Our management has significant flexibility in applying the net proceeds we expect to receive from the issuance of ordinary shares under our ATM program. We intend to use the net proceeds from this offering for general corporate purposes, which may include capital expenditures, working capital and general and administrative expenses. However, because the net proceeds are not required to be allocated to any specific investment or transaction, investors cannot determine at the time of issuance the value or propriety of our application of the net proceeds, and investors may not agree with our decisions. In addition, our use of the net proceeds from the offering may not yield a significant return or any return at all. The failure by our management to apply these funds effectively could have an adverse effect on our financial condition, results of operations or the trading price of our ordinary shares.

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

As of September 30, 2017, we had $20.0 million of outstanding borrowings under our Loan and Security Agreement with Hercules, which we are required to repay in monthly principal installments from December 2018 through May 2020. We could in the future incur additional debt obligations beyond our borrowings from Hercules. Our existing loan obligations, together with other similar obligations that we may incur in the future, could have significant adverse consequences, including:

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

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

Risks Related to Other Legal Compliance Matters

Our relationships with customers and third-party payers will be subject to applicable anti-kickback, anti-bribery, fraud and abuse and other 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 payers 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 payers and customers may expose us to broadly applicable anti-bribery laws, including the Foreign Corrupt Practices Act, as well as 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.

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. 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 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 could cause us to incur substantial liabilities and to limit commercialization of our therapies.

We face an inherent risk of product liability related to the testing of our product candidates in human clinical trials and in connection with product sales. 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.

Dependent upon the country where the clinical trial is conducted, we currently hold a maximum of €6,000,000 and minimum of €2,000,000 in clinical trial insurance coverage in the aggregate, with a per incident limit of €450,000 to €1,000,000 with respect to the clinical studies we conduct. Such coverage may not be adequate to cover all liabilities that we may incur. We may need to increase our insurance coverage as we expand our clinical trials. In addition, insurance coverage is increasingly expensive. We may not be able to maintain insurance coverage at a reasonable cost or in an amount adequate to satisfy any liability that may arise.

Risks Related to Employee Matters and Managing Growth

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

We are highly dependent on hiring, training, retaining and motivating key personnel to lead our research and development, clinical operations and manufacturing efforts. Although we have entered into employment agreements with our key personnel, each of them may terminate their employment on short notice. We do not maintain key person insurance for any of our senior management or employees.

The loss of the services of our key employees could impede the achievement of our research and development 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 because of the limited number of individuals in our industry with the breadth and depth of skills and experience required to successfully develop 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.

Risks Related to Our Ordinary Shares

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

Our share price has been and may in the future be volatile. From the start of trading of our ordinary shares on the NASDAQ Global Select Market on February 4, 2014 through October 31, 2017, the sale price of our ordinary shares ranged from a high of $36.38 to a low of $4.72. The closing price on October 30, 2017, was $15.42 per ordinary share. The stock market in general and the market for smaller biopharmaceutical companies in particular, have experienced extreme volatility that has often been unrelated to the operating performance of particular companies. The market price for our ordinary shares may be influenced by many factors, including:

- the success of competitive products or technologies;
- results of clinical trials of our product candidates or those of our competitors;
- public perception of gene therapy;
- regulatory delays and greater government regulation of potential products due to adverse events;
- regulatory or legal developments in the European Union, the United States and other countries;
- developments or disputes concerning patent applications, issued patents or other proprietary rights;
- the recruitment or departure of key personnel;
- the level of expenses related to any of our product candidates or clinical development programs;
- the results of our efforts to discover, develop, acquire or in- license additional product candidates or products;
- actual or anticipated changes in estimates as to financial results, development timelines or recommendations by securities analysts;
- variations in our financial results or those of companies that are perceived to be similar to us;
- changes in the structure of healthcare payment systems;
- market conditions in the pharmaceutical and biotechnology sectors; and
- general economic, industry and market conditions.

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

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

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

Our directors, named executive offices and major shareholders holding more than 5% of our outstanding ordinary shares, in the aggregate, beneficially own approximately 31.2% of our issued shares as at September 30, 2017. As a result, if these shareholders were to choose to act together, they may be able, as a practical matter, to control many matters submitted to our shareholders for approval, as well as our management and affairs. For example, these persons, if they choose to act together, could control the election of the board directors and the approval of any merger, consolidation or sale of all or substantially all 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 our 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 board. These provisions include:

- staggered terms of our directors;
- a provision that our 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 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 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. Accordingly, shareholders cannot rely on dividend income from our ordinary shares and any returns on an investment in our ordinary shares will likely depend entirely upon any future appreciation in the price of our ordinary shares.

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

We are an emerging growth company, as defined in the Jumpstart Our Business Startups Act of 2012 (“JOBS Act”) and may remain an emerging growth company for up to five years from our initial public offering. 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.

If some investors find our ordinary shares less attractive because of our reliance on these exemptions, trading market for our ordinary shares may be less active and our share price may be more volatile.

We ceased to qualify as a foreign private issuer as of January 1, 2017, and therefore must comply with the Exchange Act, which will result in additional legal, accounting and other expenses.

Beginning in January 2017, we must comply with the Exchange Act reporting and other requirements applicable to U.S. domestic filers, which are more detailed and extensive than the requirements for foreign private issuers to which we were previously subject. In addition, we are now required to report our financial results under U.S. GAAP, including our historical financial results, which have previously been prepared in accordance with International Financial Reporting Standards (“IFRS”). We have made changes in our corporate governance practices in accordance with various SEC and NASDAQ rules. The transition to U.S. GAAP reporting has required additional expenditures, and the related regulatory, compliance and insurance costs to us may be significantly higher than the costs we incurred as a foreign private issuer.

If we fail to 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.

If we fail to maintain the adequacy of our internal control over financial reporting, we may not be able to conclude on an ongoing basis that we have effective internal control over financial reporting. If we fail to maintain effective internal control over financial reporting, 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. Our reporting and compliance obligations may place a significant strain on our management, operational and financial resources and systems for the foreseeable future.

Risks for U.S. Holders

We qualify as a passive foreign investment company as of December 31, 2016 and may qualify as a passive foreign investment company in the future, which may result in adverse U.S. federal income tax consequence to U.S. holders.

Based on our average value of our gross assets, our cash and cash equivalents as well as the price of our shares we qualify as a passive foreign investment company ("PFIC") for U.S. federal income tax for 2016. 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 to produce 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 continue to qualify as a PFIC in future taxable years. 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 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 the U.S. holder disposed of any ordinary shares or received any distributions in respect of ordinary shares during such year.

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

Although we now report as a U.S. domestic filer for SEC reporting purposes, we are incorporated under the laws of the Netherlands. Some of the members of our board 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 Board members 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. To obtain a judgment which is enforceable in the Netherlands, the party in whose favor a final and conclusive judgment of the U.S. court has been rendered will be required to file its claim with a court of competent jurisdiction in the Netherlands. Such party may submit to the Dutch court the final judgment rendered by the U.S. court. If and to the extent that the Dutch court finds that the jurisdiction of the U.S. court has been based on grounds which are internationally acceptable and that proper legal procedures have been observed, the Dutch court will, in principle, give binding effect to the judgment of the U.S. court, unless such judgment contravenes principles of public policy of the Netherlands. Dutch courts may deny the recognition and enforcement of punitive damages or other awards. Moreover, a Dutch court may reduce the amount of damages granted by a U.S. court and recognize damages only to the extent that they are necessary to compensate actual losses or damages. Enforcement and recognition of judgments of U.S. courts in the Netherlands are solely governed by the provisions of the Dutch Civil Procedure Code.

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

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

Although we now report as a U.S. domestic filer for SEC purposes, our corporate affairs are governed by our articles of association and by the laws governing companies incorporated in the Netherlands. The rights of our shareholders and the responsibilities of members of our board under Dutch law are different than under the laws of some U.S. jurisdictions. In the performance of their duties, our board members are required by Dutch law to consider the interests of uniQure, its shareholders, its employees and other stakeholders and not only those of our shareholders.

Item 2. Unregistered Sales of Equity Securities and Use of Proceeds

None.

Item 3. Defaults Upon Senior Securities

None.

Item 4. Mine Safety Disclosures

Not applicable.

Item 5. Other Information

None.

Item 6. Exhibits

See the Exhibit Index immediately preceding the signature page to this Quarterly Report on Form 10-Q for a list of exhibits filed or furnished with this report, which Exhibit Index is incorporated herein by reference.

EXHIBIT INDEX

3.1	Amended Articles of Association (incorporated by reference to Exhibit 1.1 of the Company's annual report on Form 10-K for the year ended December 31, 2016 (file no. 0001-36294) filed with the Securities and Exchange Commission).
10.1*	Letter agreement dated October 26, 2017 between uniQure N.V. and Matthew Kapusta, amending Mr. Kapusta's employment agreement dated December 9, 2014, as amended.
31.1*	Rule 13a-14(a)/15d-14(a) Certification of Principal Executive Officer
31.2*	Rule 13a-14(a)/15d-14(a) Certification of Principal Financial Officer
32.1±	Section 1350 Certification of Principal Executive Officer and Principal Financial Officer
101*	The following financial information from our Quarterly Report on Form 10-Q for the period ended September 30, 2017, filed with the Securities and Exchange Commission on August 8, 2017 is formatted in Extensible Business Reporting Language ("XBRL"): (i) Consolidated Balance Sheets; (ii) Consolidated Statements of Operations and Comprehensive Loss; (iii) Consolidated Statements of Shareholders' Equity; (iv) Consolidated Statements of Cash Flows; and (v) Notes to Consolidated Financial Statements (tagged as blocks of text)

* Filed herewith.

± Furnished herewith

SIGNATURE

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

UNIQURE, N.V.

By: /s/ Matthew Kapusta
Matthew Kapusta
Chief Executive Officer
(Principal Executive Officer and Principal Financial Officer)

By: /s/ Christian Klemt
Christian Klemt
Chief Accounting Officer

Dated November 1, 2017

Exhibit 10.1

October 26, 2017

Matthew Kapusta
38 Devon Road
Chestnut Hill, MA 02467

Dear Matt:

We refer to the employment agreement dated December 9, 2014, between uniQure, Inc. (together with all of its affiliates, the "Company") and you (the "Employment Agreement"), by which you have served as Chief Financial Officer of the Company; to the letter agreement dated October 19, 2016 setting out your compensation arrangements in the role of interim Chief Executive Officer; and to the amendment to your Employment Agreement dated March 14, 2017 setting out your compensation arrangements in the role as Chief Executive Officer.

The Board of Directors of uniQure N.V. (the "Board") is pleased to offer you the following amendments and supplements to your Employment Agreement (all terms used but not defined herein shall be as defined in the Employment Agreement):

1. Section 9.6 (c) of the Employment Agreement shall be replaced in its entirety with the following:

   "*Separation Benefits*.

   c. Should Executive experience a termination of employment during the Employment Period pursuant to Section 9.1(i), then, in addition to the Accrued Benefits, Executive shall be entitled to:

      1. a lump sum payment equal to two times Executive's then-current Base Salary (less necessary withholdings and authorized deductions) to be paid no later than sixty (60) days after the termination date;

      2. the Continuation Health Benefit as set out in 9.6.b(2), except that the term shall be extended for eighteen (18) months, rather than twelve (12);

      3. a Bonus paid at target for the year of termination, as set forth in and subject to Section 4.3, pro-rated in relation to the Executive's termination date;

      4. a lump sum representing two times the Executive's Bonus paid at target as set forth in and subject to Section 4.3 to be paid no later than sixty (60) days after the termination date; and

      5. accelerated vesting of any and all equity awards which remain unvested as of Executive's termination date.

   The Company and Executive agree that any severance payments provided for in this section 9.6 do not result in extending employment beyond the termination date."

All payments under this agreement shall be made subject to applicable tax withholding, and the Company shall withhold from any payments under this agreement all federal, state, and local taxes as the Company is required to withhold pursuant to any law or governmental rule or regulation. You shall be solely responsible for all federal, state, and local taxes due with respect to any payment received under this agreement or otherwise in connection with your employment.

With the exception of the changes stated above, the terms and conditions of employment set out in your Employment Agreement remain the same. This letter shall form a part of the Employment Agreement and shall be governed by the terms of the Employment Agreement.

Best regards,

/s/ Philip Astley-Sparke

Philip Astley-Sparke
Chairman of the Board of Directors

This letter, the letter dated March 14, 2017, and my Employment Agreement, together, constitute the entire agreement between the Company and me with respect to my employment with the Company and may not be altered or amended unless in writing and signed by both parties.

/s/ Matthew Kapusta
Matthew Kapusta

October 26, 2017
Date

Exhibit 31.1

Certification of Chief Executive Officer

I, Matthew Kapusta, certify that:

1. I have reviewed this Quarterly Report on Form 10-Q of uniQure N.V.;

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 registrant as of, and for, the periods presented in this report;

4. The registrant's other certifying officer and I are responsible for establishing and maintaining disclosure controls and procedures (as defined in Exchange Act Rules 13a-15(e) and 15d-15(e)) and internal controls over financial reporting (as defined in Exchange Act Rules 13a-15(f) and 15d-15(f)) for the registrant 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 registrant, including its consolidated subsidiaries, is made known to us by others within those entities, particularly during the period in which this report is being prepared;

(b) Designed such internal control over financial reporting, or caused such internal control over financial reporting to be designed under our supervision, to provide reasonable assurance regarding the reliability of financial reporting and the preparation of financial statements for external purposes in accordance with generally accepted accounting principles;

(c) Evaluated the effectiveness of the registrant's disclosure controls and procedures and presented in this report our conclusions about the effectiveness of the disclosure controls and procedures, as of the end of the period covered by this report based on such evaluation;

(d) Disclosed in this report any change in the registrant's internal control over financial reporting that occurred during the registrant's most recent fiscal quarter (the registrant's fourth fiscal quarter in the case of an annual report) that has materially affected, or is reasonably likely to materially affect, the registrant's internal control over financial reporting;

5. The registrant's other certifying officer and I have disclosed, based on our most recent evaluation of internal control over financial reporting, to the registrant's auditors and the audit committee of the registrant'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 registrant'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 registrant's internal control over financial reporting.

By: /s/ MATTHEW KAPUSTA

Matthew Kapusta
Chief Executive Officer
November 1, 2017

Exhibit 31.2

Certification of Chief Financial Officer

I, Matthew Kapusta, certify that:

1. I have reviewed this Quarterly Report on Form 10-Q of uniQure N.V.;

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 registrant as of, and for, the periods presented in this report;

4. The registrant's other certifying officer and I are responsible for establishing and maintaining disclosure controls and procedures (as defined in Exchange Act Rules 13a-15(e) and 15d-15(e)) and internal controls over financial reporting (as defined in Exchange Act Rules 13a-15(f) and 15d-15(f)) for the registrant 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 registrant, including its consolidated subsidiaries, is made known to us by others within those entities, particularly during the period in which this report is being prepared;

(b) Designed such internal control over financial reporting, or caused such internal control over financial reporting to be designed under our supervision, to provide reasonable assurance regarding the reliability of financial reporting and the preparation of financial statements for external purposes in accordance with generally accepted accounting principles;

(c) Evaluated the effectiveness of the registrant's disclosure controls and procedures and presented in this report our conclusions about the effectiveness of the disclosure controls and procedures, as of the end of the period covered by this report based on such evaluation;

(d) Disclosed in this report any change in the registrant's internal control over financial reporting that occurred during the registrant's most recent fiscal quarter (the registrant's fourth fiscal quarter in the case of an annual report) that has materially affected, or is reasonably likely to materially affect, the registrant's internal control over financial reporting;

5. The registrant's other certifying officer and I have disclosed, based on our most recent evaluation of internal control over financial reporting, to the registrant's auditors and the audit committee of the registrant'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 registrant'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 registrant's internal control over financial reporting.

By: /s/ MATTHEW KAPUSTA

Matthew Kapusta
Chief Financial Officer
November 1, 2017

Exhibit 32.1

CERTIFICATION PURSUANT TO 18 U.S.C. SECTION 1350
AS ADOPTED PURSUANT TO SECTION 906 OF THE SARBANES-OXLEY ACT OF 2002

In connection with this Quarterly Report of uniQure N.V. (the "Company") on Form 10-Q for the period ended September 30, 2017, as filed with the Securities and Exchange Commission on the date hereof (the "Report"), the undersigned, Matthew Kapusta, Chief Executive Officer and Chief Financial Officer of the Company, hereby certifies, pursuant to 18 U.S.C. Section 1350, as adopted pursuant to Section 906 of the Sarbanes-Oxley Act of 2002, that:

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.

By: /s/ MATTHEW KAPUSTA

Matthew Kapusta
Chief Executive Officer and
Chief Financial Officer
November 1, 2017

A signed original of this written statement required by Section 906 has been provided to uniQure N.V. and will be retained by uniQure N.V. and furnished to the SEC or its staff upon request.