
**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 June 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 (State or other jurisdiction of incorporation or organization)	Not applicable (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 <input type="checkbox"/>	Accelerated filer <input checked="" type="checkbox"/>
Non accelerated filer (do not check if smaller reporting company) <input type="checkbox"/>	Smaller reporting company <input type="checkbox"/>
Emerging growth company <input checked="" type="checkbox"/>	

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 August 3, 2017, the registrant had 25,629,099 shares of common shares, par value €0.05, outstanding.

TABLE OF CONTENTS

	<u>Page</u>
<u>PART I – FINANCIAL INFORMATION</u>	
<u>Item 1</u>	<u>Financial Statements</u>
	4
<u>Item 2</u>	<u>Management’s Discussion and Analysis of Financial Condition and Results of Operations</u>
	23
<u>Item 3</u>	<u>Quantitative and Qualitative Disclosures About Market Risk</u>
	36
<u>Item 4</u>	<u>Controls and Procedures</u>
	36
<u>PART II – OTHER INFORMATION</u>	
<u>Item 1</u>	<u>Legal Proceedings</u>
	37
<u>Item 1A</u>	<u>Risk Factors</u>
	37
<u>Item 2</u>	<u>Unregistered Sales of Equity Securities and Use of Proceeds</u>
	57
<u>Item 3</u>	<u>Defaults Upon Senior Securities</u>
	57
<u>Item 4</u>	<u>Mine Safety Disclosures</u>
	57
<u>Item 5</u>	<u>Other Information</u>
	57
<u>Item 6</u>	<u>Exhibits</u>
	57

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 by the use of 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, or SEC, 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 June 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 of 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 CONDENSED CONSOLIDATED BALANCE SHEETS

	June 30, 2017	December 31, 2016
	in thousands, except share and per share amounts	
Current assets		
Cash and cash equivalents	\$ 104,087	\$ 132,496
Accounts receivable and accrued income	4,190	3,680
Accounts receivable and accrued income from related party	1,627	5,500
Prepaid expenses	889	996
Other current assets	632	1,274
Total current assets	111,425	143,946
Non-current assets		
Property, plant and equipment, net	35,410	35,702
Intangible assets, net	8,746	8,324
Goodwill	505	465
Other non-current assets	1,879	1,828
Total non-current assets	46,540	46,319
Total assets	\$ 157,965	\$ 190,265
Current liabilities		
Accounts payable	\$ 3,458	\$ 5,524
Accrued expenses and other current liabilities	9,440	9,766
Current portion of long-term debt	4,319	605
Current portion of deferred rent	710	684
Current portion of deferred revenue	5,203	6,142
Total current liabilities	23,130	22,721
Non-current liabilities		
Long-term debt, net of current portion	16,153	19,631
Deferred rent, net of current portion	8,494	6,781
Deferred revenue, net of current portion	78,728	75,612
Contingent consideration	2,415	1,838
Other non-current liabilities	1,759	51
Total non-current liabilities	107,549	103,913
Total liabilities	130,679	126,634
Commitments and contingencies (see note 13)		
Shareholders' equity		
Common shares, €0.05 par value: 60,000,000 shares authorized at June 30, 2017 and December 31, 2016 and 25,620,073 and 25,257,420 shares issued and outstanding at June 30, 2017 and December 31, 2016, respectively.	1,612	1,593
Additional paid-in-capital	469,104	464,653
Accumulated other comprehensive loss	(5,831)	(6,557)
Accumulated deficit	(437,599)	(396,058)
Total shareholders' equity	27,286	63,631
Total liabilities and shareholders' equity	\$ 157,965	\$ 190,265

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

uniQure N.V.

UNAUDITED CONDENSED CONSOLIDATED STATEMENTS OF OPERATIONS AND
COMPREHENSIVE LOSS

	Three months ended June 30,		Six months ended June 30,	
	2017	2016	2017	2016
	in thousands, except share and per share amounts			
License revenues	\$ (226)	\$ 250	\$ 8	\$ 491
License revenues from related party	987	1,006	1,936	1,984
Collaboration revenues	3,058	1,558	4,638	2,984
Collaboration revenues from related party	1,123	1,637	1,681	3,287
Total revenues	4,942	4,451	8,263	8,746
Operating expenses:				
Research and development expenses	(16,866)	(19,221)	(33,860)	(35,927)
Selling, general and administrative expenses	(5,410)	(7,834)	(11,768)	(15,132)
Total operating expenses	(22,276)	(27,055)	(45,628)	(51,059)
Other income	266	475	582	920
Other expense	(2,640)	—	(2,640)	—
Loss from operations	(19,708)	(22,129)	(39,423)	(41,393)
Interest income	12	15	23	37
Interest expense	(502)	(549)	(1,006)	(1,178)
Foreign currency gains / (losses), net	(1,071)	920	(1,164)	(1,268)
Other non-operating income, net	—	330	29	647
Loss before income tax expense	(21,269)	(21,413)	(41,541)	(43,155)
Income tax benefit / (expense)	—	333	—	(224)
Net loss	\$ (21,269)	\$ (21,080)	\$ (41,541)	\$ (43,379)
Other comprehensive loss, net of income tax:				
Foreign currency translation adjustments net of tax impact of nil and \$0.3 million for the three months ended June 30, 2017 and 2016, respectively, and nil and (0.2) million for the six months ended June 30, 2017 and 2016, respectively.	404	(4,101)	726	579
Total comprehensive loss	\$ (20,865)	\$ (25,181)	\$ (40,815)	\$ (42,800)
Basic and diluted net loss per common share	\$ (0.83)	(0.84)	(1.63)	(1.74)
Weighted average shares used in computing basic and diluted net loss per common share	25,560,348	25,077,350	25,502,301	24,886,996

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

uniQure N.V.

UNAUDITED CONDENSED CONSOLIDATED STATEMENTS OF SHAREHOLDERS' EQUITY

	Common 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	—	—	—	—	(41,541)	(41,541)
Other comprehensive income	—	—	—	726	—	726
Exercise of share options	279,153	15	924	—	—	939
Shares distributed during the period	83,500	4	(4)	—	—	—
Share-based compensation expense	—	—	3,531	—	—	3,531
Balance at June 30, 2017	25,620,073	\$ 1,612	\$ 469,104	\$ (5,831)	\$ (437,599)	\$ 27,286

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

uniQure N.V.

UNAUDITED CONDENSED CONSOLIDATED STATEMENTS OF CASH FLOWS

	<u>Six months ended June 30,</u>	
	<u>2017</u>	<u>2016</u>
	in thousands	
Cash flows from operating activities		
Net loss	\$ (41,541)	\$ (43,379)
Adjustments to reconcile net loss to net cash used in operating activities:		
Depreciation, amortization and impairments	4,257	2,989
Share-based compensation expense	3,531	4,097
Change in fair value of derivative financial instruments	397	(393)
Unrealized foreign exchange results	1,168	1,303
Change in deferred taxes	-	224
Change in lease incentive	1,634	(306)
Changes in operating assets and liabilities:		
Accounts receivable, prepaid expenses and other current assets	5,096	(1,307)
Inventories	-	80
Accounts payable	(1,334)	(1,868)
Accrued expenses and other liabilities	(696)	2,982
Deferred revenue	(3,315)	(2,891)
Net cash used in operating activities	<u>(30,803)</u>	<u>(38,469)</u>
Cash flows from investing activities		
Restricted cash	-	(617)
Purchase of intangible assets	(578)	-
Purchase of property, plant and equipment	(2,830)	(3,553)
Net cash used in investing activities	<u>(3,408)</u>	<u>(4,170)</u>
Cash flows from financing activities		
Proceeds from issuance of shares	939	2,195
Repayment of capital lease obligations	-	(98)
Net cash generated from financing activities	<u>939</u>	<u>2,097</u>
Currency effect cash and cash equivalents	4,863	2,848
Net increase / (decrease) in cash and cash equivalents	<u>(28,409)</u>	<u>(37,694)</u>
Cash and cash equivalents at beginning of period	132,496	221,626
Cash and cash equivalents at the end of period	<u>\$ 104,087</u>	<u>\$ 183,932</u>
Supplemental cash flow disclosures:		
Cash paid for interest	\$ 840	\$ 1,118
Non-cash adjustments in purchases of intangible assets and property, plant and equipment	\$ (1,108)	\$ 2,218

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

1 General business information

uniQure N.V. (“uniQure” or the “Company”) was founded in 1998 by scientists at the Academic Medical Center of the University of Amsterdam. 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 initially operated through its predecessor company, Amsterdam Molecular Therapeutics Holding N.V. (“AMT”). The Company was incorporated in January 2012 to acquire and continue the gene therapy business of AMT. Effective February 10, 2014, in connection with its initial public offering, the Company converted into a public company with limited liability and changed its legal name from uniQure B.V. to uniQure N.V. The Company is registered with the Dutch Trade Register of the Chamber of Commerce (“*handelsregister van de Kamer van Koophandel en Fabrieken*”) in Amsterdam, the Netherlands under number 54385229. The Company’s headquarters is 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.

Effective January 1, 2017, the Company ceased to qualify as a foreign private issuer. 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 common stock is listed on the NASDAQ Global Market and trades under the symbol “QURE”.

2 Summary of significant accounting policies

2.1 Basis of preparation

The Company prepared these unaudited condensed 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 condensed 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 condensed 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.

The year-end condensed balance sheet data was derived from audited financial statements, but does not include all disclosures required by U.S. GAAP. Certain information and footnote disclosures normally included in financial statements prepared in accordance with U.S. GAAP have been condensed or omitted. The condensed results of operations for the six months ended June 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 condensed 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 condensed 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 six months ended June 30, 2017.

2.5 Recent accounting pronouncements

There have been no new accounting pronouncements or changes to accounting pronouncements during the six months ended June 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 one 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 as a result 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.

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 has the ability to 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 June 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	<u>132,496</u>	<u>—</u>	<u>—</u>	<u>132,496</u>	
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	<u>\$ —</u>	<u>\$ —</u>	<u>\$ 1,900</u>	<u>\$ 1,900</u>	
At June 30, 2017					
Assets:					
Cash and cash equivalents	\$ 104,087	\$ —	\$ —	\$ 104,087	
Total assets	<u>104,087</u>	<u>—</u>	<u>—</u>	<u>104,087</u>	
Liabilities:					
Derivative financial instruments - debt	—	—	3	3	Accrued expenses and other current liabilities
Derivative financial instruments - related party	—	—	34	34	Other non-current liabilities
Contingent consideration	—	—	2,415	2,415	
Total liabilities	<u>\$ —</u>	<u>\$ —</u>	<u>\$ 2,452</u>	<u>\$ 2,452</u>	

Changes in Level 3 items during the six months ended June 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	415	(31)	384
Currency translation effects	162	6	168
Balance at June 30, 2017	\$ 2,415	\$ 37	\$ 2,452

	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	254	(647)	(393)
Currency translation effects	57	18	75
Balance at June 30, 2016	\$ 3,237	\$ 208	\$ 3,445

Contingent consideration

In connection with the Company's acquisition of InoCard GmbH in 2014, the Company recorded contingent consideration related to amounts potentially payable to InoCard's former shareholders. The amounts payable are contingent upon realization of the following milestones:

- Successful completion of GLP safety and toxicology study;
- First patient dosed in a clinical study; and
- Full proof-of-concept of the product in human patients after finalization of a phase I/II 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 two 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. 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, next to the passing of time, the unobservable inputs results in the following fair value changes:

	June 30, 2017 in thousands
Change in fair value	
Moving out of all milestones by 6 months	\$ (152)
Increasing the POS for the first milestone by 20%	792
Decreasing the POS for the first milestone by 20%	(792)
Reducing the discount rate from 14.5% to 10%	312
Increasing the discount rate from 14.5% to 20%	(282)

	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 June 30, 2017, was \$0.0 million (December 31, 2016: \$0.1 million), and are described in more detail below.

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

BMS collaboration

On April 6, 2015, the Company entered into a number of 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 common 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 common 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 through 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 six months ended June 30, 2017, the Company generated all of its 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 will hold the global rights to the development of the hemophilia B program and will not be required to provide any future 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 (exceeding 5%).

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

	<u>Three months ended June 30,</u>		<u>Six months ended June 30,</u>	
	<u>2017</u>	<u>2016</u>	<u>2017</u>	<u>2016</u>
	in thousands			
Bristol Myers Squibb	\$ 2,110	\$ 2,643	\$ 3,617	\$ 5,271
Chiesi Farmaceutici S.p.A	2,832	1,808	4,646	3,475
Total	\$ 4,942	\$ 4,451	\$ 8,263	\$ 8,746

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

	<u>June 30,</u>	<u>December 31,</u>
	<u>2017</u>	<u>2016</u>
	in thousands	
Bristol Myers Squibb	\$ 1,627	\$ 5,500
Chiesi Farmaceutici S.p.A	4,190	3,680
Total	\$ 5,817	\$ 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) 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 and when 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 six months ended June 30, 2017.

The Company recognized \$1.0 million and \$1.9 million of license revenue for the three and six months ended June 30, 2017, respectively, and compared to \$1.0 million and \$2.0 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 \$1.7 million of collaboration revenue during the three and six months ended June 30, 2017, respectively, compared to \$1.6 million and \$3.3 million during the same periods in 2016.

Manufacturing revenue – BMS

BMS and the Company also entered into a 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 commercial product to BMS.

Chiesi collaboration

In 2013, the Company entered into two agreements with Chiesi, a family-owned Italian pharmaceutical company, 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.

The Company had evaluated the Chiesi Agreements and had determined that they were a revenue arrangement with multiple elements. The Company’s substantive deliverables under the Chiesi Agreements included an exclusive license to its technology, research and development services, and commercial manufacturing. The Company concluded that the Chiesi Agreements consisted of three units of accounting, including (i) co-development and active contribution to the collaboration by providing technology access and know-how in the field of gene therapy, (ii) provision of employees, goods and services for research and development activities and (iii) commercial manufacturing.

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. The settlement terms require the Company to repay €2.0 million (\$2.3 million) in up-front payments received in 2013.

In July 2017, the parties terminated the Hemophilia Collaboration Agreement and the Company reacquired rights associated with its hemophilia B program in Europe and certain other territories (see note 14).

License revenue – Chiesi

Upon the closing of the Chiesi Agreements on June 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.

The Company recognized \$(0.2) million and \$0.0 million of license revenue during the three and six months ended June 30, 2017, respectively, compared to \$0.2 million and \$0.5 million during the same periods in 2016. The Company recognized the license revenue for the three and six months ended June 30, 2017, net of a \$0.5 million reduction for amounts previously amortized in relation to the \$2.3 million up-front payments that the Company will be required to repay in accordance with the Glybera Termination Agreement. The Company also reduced the deferred revenue balance as of June 30, 2017, by \$1.8 million for license revenue not yet earned.

Collaboration revenue – Chiesi

Prior to the termination of the Hemophilia Collaboration Agreement, 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 \$3.1 million and \$4.6 million of collaboration revenue from the co-development of hemophilia B during the three and six months ended June 30, 2017, respectively, compared to \$1.6 million and \$3.0 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 June 30, 2017, and December 31, 2016:

	June 30, 2017	December 31, 2016
	in thousands	
Leasehold improvements	\$ 32,405	\$ 30,582
Laboratory equipment	15,507	14,166
Office equipment	2,870	2,710
Construction-in-progress	376	313
Total property, plant, and equipment	51,158	47,771
Less accumulated depreciation	(15,748)	(12,069)
Property, plant and equipment, net	\$ 35,410	\$ 35,702

Total depreciation expense was \$1.7 million and \$3.4 million during the three and six months ended June 30, 2017, respectively, compared to \$1.3 million and \$2.7 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:

	June 30, 2017	December 31, 2016
	in thousands	
Licenses	\$ 9,058	\$ 7,799
Acquired research & development	5,330	4,908
Total intangible assets	14,388	12,707
Less accumulated amortization and impairment	(5,642)	(4,383)
Intangible assets, net	\$ 8,746	\$ 8,324

Amortization expense was \$0.7 million and \$0.8 million for the three and six months ended June 30, 2017, respectively, compared to \$0.2 million and \$0.3 million during the same periods in 2016. All amortization, with the exception of \$0.6 million related to the termination of the Chiesi collaboration, which was presented in other expense in the three and six months ended June 30, 2017, were included in research and development expenses.

7 Accrued expenses and other current liabilities / other non-current liabilities

Accrued expenses and other current liabilities include the following items:

	June 30, 2017	December 31, 2016
	in thousands	
Accruals for services provided by vendors-not yet billed	\$ 3,092	\$ 3,824
Personnel related accruals and liabilities	3,974	5,559
Other current liabilities	2,374	383
Total	\$ 9,440	\$ 9,766

Other current liabilities as at June 30, 2017 include accrued rent for the vacated facilities at the Company's previous site at the Academische Medisch Centrum Amsterdam (see note 12) as well as the current portion of liabilities related to the Glybera withdrawal (see below).

Restructuring plan

In November 2016, the Company announced a plan to restructure its activities as a result of 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. In December 2016, the Company accrued \$1.1 million of termination benefits associated with the restructuring. At various dates during the six months ended June 30, 2017, the Company entered into termination agreements with additional employees and recognized related termination costs of \$0.9 million for services rendered by these employees during 2017. The change in the accrual of termination benefits (recognized within research and development expenses) for the six months ended June 30, 2017 was:

	Accrued termination benefits in thousands
Balance at December 31, 2016	\$ 1,148
Accrued through profit and loss	908
Payments	(1,435)
Currency translation effects	48
Balance at June 30, 2017	\$ 669

Other non-current liabilities

As part of the Glybera Termination Agreement, the Company agreed to repay €2.0 million (\$2.3 million) of the upfront payment it received upon entering into the initial agreement in 2013. The amounts will be payable in installments through January 2019. As of June 30, 2017, the Company accrued €1.5 million (\$1.7 million), of which €0.5 million (\$0.6 million) is included within other current liabilities. The Company may owe up to an additional €1.8 million (\$2.0 million) depending on the number of patients treated with Glybera prior to its withdrawal. As of June 30, 2017, the Company accrued no costs based on its best estimate of payments it will be required to be made. See also note 14 in relation to the terms of the Chiesi collaboration.

According to the Glybera Termination Agreement the Company will be responsible for terminating the Phase IV post-approval study and accrued \$0.9 million (presented as other expenses) in relation to such costs as of June 30, 2017, of which \$0.6 million is included within other current liabilities.

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 June 30, 2018, to May 1, 2020. As at June 30, 2017, and December 31, 2016, \$20.0 million were 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 Loan, was \$20.5 million as of June 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 six months ended June 30, 2017, was \$1.3 million and \$1.6 million, respectively. The Company recognized a foreign currency loss of \$0.7 million and a gain of \$0.2 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 six months ended June 30, 2017, was \$0.5 million and \$1.0 million, respectively, compared to \$0.6 million and \$1.2 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 Share-based compensation

The Company recognized share-based compensation expense totaling \$1.9 million and \$3.5 million during the three and six months ended June 30, 2017, respectively, compared to \$1.5 million and \$4.1 million during the same periods in 2016.

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

	Three months ended June 30,		Six months ended June 30,	
	2017	2016	2017	2016
	in thousands			
Research and development - employees	\$ 794	\$ 918	\$ 1,481	\$ 1,777
Selling, general and administrative - employees	1,132	531	2,050	1,650
Research and development - non-employees	—	—	—	670
Total	\$ 1,926	\$ 1,449	\$ 3,531	\$ 4,097

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

Award type	Three months ended June 30,		Six months ended June 30,	
	2017	2016	2017	2016
	in thousands			
Share options	\$ 883	\$ 1,306	\$ 1,709	\$ 3,690
Restricted share units ("RSUs")	716	82	1,232	235
Performance share units ("PSUs")	327	61	590	172
Total	\$ 1,926	\$ 1,449	\$ 3,531	\$ 4,097

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

	Unrecognized compensation costs	Weighted-average remaining period for recognition (in years)
Award type	in thousands	
Share options	\$ 7,520	2.74
Restricted share units	4,169	1.87
Performance share units	2,556	2.39
Total	\$ 14,245	2.42

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 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 common 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 six months ended June 30, 2017:

	2014 plan	
	Options	Weighted average exercise price
Outstanding at December 31, 2016	1,812,766	\$ 12.47
Granted	714,600	\$ 5.59
Forfeited	(118,739)	\$ 9.76
Expired	(55,764)	\$ 15.42
Outstanding at June 30, 2017	2,352,863	\$ 10.45
Fully vested and exercisable	816,586	\$ 12.93
Outstanding and expected to vest	1,536,277	\$ 9.12

During the six months ended June 30, 2017, the Company granted options to purchase an aggregate of 714,600 common shares with a total weighted-average grant date fair value of \$2.3 million, including an option to purchase 175,000 common shares granted to the Company's Chief Executive Officer (vesting equally over four years from the date of grant) and options to purchase an aggregate of 80,000 common shares granted to the Company's non-executive directors (vesting 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:

Assumptions	Three months ended June 30,		Six months ended June 30,	
	2017	2016	2017	2016
Expected volatility	75%	75%	75%	75%
Expected terms (in years)	10 years	10 years	10 years	10 years
Risk free interest rate	2.43%	1.58% - 1.96%	2.43% - 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 six months ended June 30, 2017:

	RSU	
	Number of shares	Weighted average grant-date fair value
Undistributed at December 31, 2016	307,063	\$ 9.11
Granted	408,350	\$ 5.83
Distributed	(25,000)	\$ 18.21
Forfeited	(2,275)	\$ 7.98
Undistributed at June 30, 2017	688,138	\$ 6.84

During the six months ended June 30, 2017, the Company granted an aggregate of 408,350 RSUs with a total weighted-average grant date fair value of \$2.4 million, including 175,000 RSUs granted to the Company's Chief Executive Officer (vesting equally over two years from the date of grant) and a total of 80,000 RSUs granted to non-executive directors (vesting one year from the date of grant).

During the six months ended June 30, 2017, the Company distributed 25,000 common shares in connection with the vesting of RSUs.

Performance Share Units (PSUs)

The following table summarizes the PSUs activity for the six months ended June 30, 2017:

	PSU	
	Number of shares	Weighted average grant-date fair value
Undistributed at December 31, 2016	111,564	\$ 5.76
Retired	(12,000)	\$ 5.76
Granted	13,000	\$ 4.99
Distributed	(58,500)	\$ 5.76
Undistributed at June 30, 2017	54,064	\$ 5.57

During the six months ended June 30, 2017, the Company awarded an aggregate of 426,250 PSUs to members of its senior management, including 162,500 PSUs to its Chief Executive Officer. As the earning of these PSUs is discretionary based on the Board's assessment of the performance through 2017, these PSUs are not included in the above table.

In September 2016, the Company awarded 61,560 PSUs to its Chief Executive Officer, subject to the successful implementation of the strategic plan. As the earning of these PSUs is discretionary based on the Board's assessment of the Chief Executive Officer's performance through 2017, these PSUs are also not included in the above table.

Other Plans

The following table summarizes option activity under the Company's Other Plans for the six months ended June 30, 2017:

	<u>Other plans</u>	
	<u>Options</u>	<u>Weighted average exercise price</u>
Outstanding at December 31, 2016	187,500	\$ 17.93
Granted	150,000	\$ 5.31
Expired	(62,500)	\$ 27.82
Outstanding at June 30, 2017	275,000	\$ 8.80
Fully vested and exercisable	31,250	\$ 12.98
Outstanding and expected to vest	243,750	\$ 8.26

Under Rule 5653(c)(4) of the NASDAQ Global Market, the Company grants share options and RSUs to certain employees as a material inducement to enter into employment with the Company. During the six months ended June 30, 2017, the Company granted 150,000 options with a weighted-average grant date fair value of \$0.5 million and 175,000 RSUs with a grant date fair value of \$1.0 million. No inducement grant options were exercised during the six months ended June 30, 2017.

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 six months ended June 30, 2017:

	<u>2012 plan</u>	
	<u>Options</u>	<u>Weighted average exercise price</u>
Outstanding at December 31, 2016	483,006	€ 5.13
Exercised	(286,304)	€ 3.07
Outstanding, fully vested and exercisable at June 30, 2017	196,702	€ 8.12

Options exercised under the 2012 plan during the six months ended June 30, 2017, resulted in total proceeds to the Company of \$0.9 million.

10 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 of 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.

11 Basic and diluted earnings per share

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

The potentially dilutive common shares are summarized below:

	June 30, 2017	June 30, 2016
	common shares	
BMS warrants	5,282,647	3,442,655
Warrants	37,175	37,175
Stock options under 2012 Plan	196,702	687,808
Stock options under 2014 Plan	2,352,863	1,749,760
Stock options (other)	275,000	1,125,000
RSUs and PSUs	742,202	235,638
Total potential dilutive common shares	8,886,589	7,278,036

12 Leases

The Company leases various office space and laboratory space under operating lease agreements, expiring at various dates through 2032. These leases are described in more detail below.

Aggregate rent expense for the three and six months ended June 30, 2017, respectively, was \$1.1 million and \$2.1 million compared to \$1.1 million and \$1.9 million in the same period during 2016, respectively. Rent expense is calculated on a straight-line basis over the term of the leases and takes into account \$11.8 million of lease incentives received.

As of June 30, 2017, aggregate minimum lease payments for the calendar years ending December 31, 2017, 2018, 2019, 2020 and 2021 and beyond were as follows:

	in thousands
2017 (six months remaining)	\$ 907
2018	3,748
2019	3,801
2020	3,854
2021 and beyond	27,781
Total minimum lease payments	\$ 40,091

Lexington, Massachusetts

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 future aggregate minimum lease payments under the non-cancellable term of the lease amount to \$13.5 million. As of June 30, 2017, the Company recorded deferred rent related to lease incentives received of \$5.9 million (December 31, 2016: \$6.2 million), with a current element of \$0.7 million (December 31, 2016: \$0.7 million). The lease provides for annual minimum increases in rent, based on a consumer price index.

Paasheuvelweg, Amsterdam

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. As a result of a downsizing of personnel in Amsterdam, the Company will seek to sublease parts of the facility. The future aggregate minimum lease payments under the non-cancellable term of the lease amount to \$26.6 million. The lease contract provides for annual minimum increases in rent, based on a consumer price index.

Meibergdreef, Amsterdam

In April 2015, uniQure entered into a lease with Jan Snel B.V. for a laboratory facility of approximately 9,300 square feet, located at the Meibergdreef in Amsterdam, the Netherlands. The minimum lease period terminates September 2018. The facility is expected to be demolished in August 2017. Accordingly, the Company accrued (recognized within other expenses) the cost to exit the lease of \$0.8 million in May 2017, when it ceased using the facility.

13 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.

14 Subsequent event

Chiesi collaboration

On July 26, 2017, the Company and Chiesi terminated their Co-Development Agreement for hemophilia B. uniQure will be responsible for all future development costs related to its hemophilia B program that would have otherwise been shared with Chiesi. The Company expects to receive approximately \$2.3 million from Chiesi in August 2017 to settle all outstanding and future amounts. The Company expects to record \$13.6 million other income during the three months ended September 30, 2017, related to the full release of the outstanding deferred revenue.

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, or 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, consistent, 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 significant business developments during the six months ended June 30, 2017:

Hemophilia B program

On January 30, 2017, we received breakthrough therapy designation from the Food and Drug Administration ("FDA") for our AMT-060 program in hemophilia B. The designation was based on results from the ongoing, dose-ranging Phase I/II study that showed sustained increases in Factor IX ("FIX"), reductions in FIX replacement usage and a near cessation of spontaneous bleeding in patients with severe disease at up to 12 months follow-up. On April 25, 2017, we received Priority Medicines ("PRIME") designation by the European Medicines Agency.

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. We will be responsible for all future development costs related to the hemophilia B program, including approximately \$3.0 million of expenses in the second half of 2017 that would have otherwise been shared with Chiesi.

We have recently made significant progress in preparing for late-stage clinical studies in hemophilia B, including the initiation of commercial-scale, GMP batches at our Lexington manufacturing facility. On March 3, 2017, the Lexington facility was recognized by Frost & Sullivan as a 2017 Manufacturing Leadership Awards winner for outstanding achievement in Engineering & Production Technology Leadership.

We anticipate meeting with U.S. and European regulators in the second half of the year to discuss our plans for a pivotal study.

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.

Glybera program

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 continue to supply product to Chiesi to treat any patients that are approved for treatment prior to October 25, 2017, and 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.

AAV 5 technology platform

On April 4, 2017, we published data demonstrating widespread transduction in the central nervous system following direct injection of our AAV5 vector in a large animal model. The method of injection used was found to result in a controlled and accurate administration with no adverse events observed in non-human primates. Varying doses of AAV5 achieved predictable transduction of connected areas of the brain. The data demonstrate that AAV5 is an effective vector for the central nervous system and has potential for the treatment of a wide range of neurological pathologies. This data will help guide the clinical development of our gene therapy product candidate AMT-130, which consists of an AAV5 vector carrying an artificial micro-RNA that silences the huntingtin gene for the treatment of Huntington's disease.

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 two clinical studies involving intravenous administration to 18 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.

Corporate developments

On January 25, 2017, we appointed Dr. Alexander Kuta as our Senior Vice President of regulatory affairs.

On June 6, 2017, we appointed Steven Zelenkofske as our Chief Medical Officer.

Financial Overview

Key components of our results of operations include the following:

	Three months ended June 30,		Six months ended June 30,	
	2017	2016	2017	2016
	in thousands			
Total revenues	\$ 4,942	\$ 4,451	\$ 8,263	\$ 8,746
Research and development expenses	(16,866)	(19,221)	(33,860)	(35,927)
Selling, general and administrative expenses	(5,410)	(7,834)	(11,768)	(15,132)
Net loss	(21,269)	(21,080)	(41,541)	(43,379)

As of June 30, 2017, and December 31, 2016, we had cash and cash equivalents of \$104.1 million and \$132.5 million, respectively. We had a net loss of \$21.3 million and \$41.5 million during the three and six months ended June 30, 2017, respectively, compared to \$21.1 million and \$43.4 million during the same periods in 2016. As of June 30, 2017, and December 31, 2016, we had accumulated deficits of \$437.6 million and \$396.1 million, respectively. We anticipate that our loss from operations will increase in the future as we:

- Advance our hemophilia B program towards late-stage clinical development. In July 2017, the Company and Chiesi terminated their Hemophilia Collaboration Agreement. Accordingly, all future development expenses will be borne by the Company (previously, Chiesi was responsible for 50% of such costs);
- Continue Investigational New Drug Application (“IND”) enabling studies for our proprietary Huntington’s disease gene therapy program and initiate a proof-of-concept clinical study;
- Advance multiple research programs related to gene therapy candidates targeting liver-directed and a central nervous system (“CNS”) 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;
- Build-out our clinical, medical and regulatory capabilities in the U.S; and
- Appoint additional executives, including our Chief Medical Officer, who was appointed in June 2017 and Chief Operating Officer, who was appointed in August 2017.

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

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. As part of our plan, we intend to eliminate between 50 and 55 net positions. In 2016, we accrued \$1.1 million related to termination benefits offered to executive employees. We also entered into termination agreements with non-executive employees in January 2017, for which we recognize the aggregated termination benefits of \$0.5 million over the relevant remaining service period during 2017. These changes are expected to reduce annual operating expenses by \$5.0 to \$6.0 million from 2018 onwards.

Following our April 2017 decision not to seek extension of our European marketing authorization for Glybera after its expiration in October 2017, we will eliminate an additional seven positions required for Glybera's manufacturing at our Amsterdam site. We expect to incur additional termination costs of \$0.3 million in 2017.

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 readily apparent 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 six months ended June 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 cost 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 (“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 and have been preparing for late-stage development. In July 2017, the Company and Chiesi terminated their Hemophilia Collaboration Agreement. Accordingly, all future development expenses will be borne by the Company (previously, Chiesi was responsible for 50% of such costs);
- AMT-130 (“Huntington’s disease”). We have incurred costs related to preclinical research for AMT-130;
- S100A1 (“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; and
- *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 of our programs.
- 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. Following the initiation of the commercialization of Glybera in September 2015, we incurred selling and marketing costs related to maintaining a patient registry and conducting a post-approval, Phase IV study for Glybera up to April 2017, when we decided to cease commercializing Glybera.

Other expense

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 the exit from our previous Amsterdam facilities.

Results of Operations

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

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

	Three months ended June 30,		
	2017	2016	2017 vs 2016
	in thousands		
Total revenues	\$ 4,942	\$ 4,451	\$ 491
Operating expenses:			
Research and development expenses	(16,866)	(19,221)	2,355
Selling, general and administrative expenses	(5,410)	(7,834)	2,424
Total operating expenses	(22,276)	(27,055)	4,779
Other income	266	475	(209)
Other expense	(2,640)	—	(2,640)
Loss from operations	(19,708)	(22,129)	2,421
Other non-operating items, net	(1,561)	716	(2,277)
Loss before income tax benefit / (expense)	(21,269)	(21,413)	144
Income tax benefit / (expense)	—	333	(333)
Net loss	\$ (21,269)	\$ (21,080)	\$ (189)

Revenue

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

	Three months ended June 30,		
	2017	2016	2017 vs 2016
	in thousands		
License revenue	\$ 761	\$ 1,256	\$ (495)
Collaboration revenue Chiesi	3,058	1,558	1,500
Collaboration revenue BMS	1,123	1,637	(514)
Total revenues	\$ 4,942	\$ 4,451	\$ 491

We expect to continue to recognize \$1.0 million in license revenue each quarter from upfront payments and target designation fees received from BMS in the second and third quarters of 2015. In association with the upfront fees received from Chiesi in 2013. We recognized (\$0.2) million license revenue during the three months ended June 30, 2017, compared to \$0.3 million during the same period in 2016. We recognized our license revenue for the three months ended June 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 will be required to repay in accordance with the Glybera Termination Agreement.

Collaboration revenue generated from research activities associated with our BMS-partnered S100A1 heart failure program during the three months ended June 30, 2017 was \$1.1 million compared to \$1.6 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 activities related to the production of preclinical material during the same period in 2016.

Collaboration revenue generated from our co-development of hemophilia B with Chiesi for the three months ended June 30, 2017, was \$3.1 million compared to \$1.6 million for the same period in 2016.

Research and development expenses

Research and development expenses for the three months ended June 30, 2017, were \$16.9 million compared to \$19.2 million for the same period in 2016.

- We incurred \$9.4 million in personnel, share-based compensation expenses and consulting cost in the three months ended June 30, 2017, compared to \$10.0 million during the three months ended June 30, 2016. The decrease was a combination of \$0.6 million one-off expenses related to termination benefits and cost reductions resulting from our restructuring initiated in November 2016;
- We incurred \$3.2 million in external services and cost related to the development of our product candidates in the three months ended June 30, 2017, compared to \$5.3 million for the same period in 2016, as we incurred lower costs in relation to the manufacturing of drug substance to supply our programs;
- We incurred \$3.9 million operating expenses and depreciation expenses related to our rented facilities in the three months ended June 30, 2017, compared to \$3.7 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 June 30, 2017, were \$5.4 million compared to \$7.8 million for the same period in 2016.

- Our expenses related to employees, contractors and consultants in the three months ended June 30, 2017, were \$2.0 million compared to \$2.2 million for the same period in 2016. The decrease was primarily driven by \$0.2 million in one-time costs related to the CEO-transition that took place in the same period in 2016;
- We incurred \$1.1 million of share-based compensation expenses in the three months ended June 30, 2017, compared to \$0.5 million for the same period in 2016. The increase was related to equity instruments offered to employees during the last twelve months;
- We incurred \$1.0 million of professional fees in the three months ended June 30, 2017, compared to \$1.3 million for the same period in 2016. We regularly incur accounting, audit and legal fees associated with operating as a public company, including the cost of our conversion from IFRS to U.S. GAAP which we initiated during the second quarter 2016;
- We incurred legal and settlement costs of \$1.9 million in connection with our arbitration proceeding with Extera during the three months ended June 30, 2016. No such costs were incurred during the three months ended June 30, 2017; and
- We incurred \$0.1 million of costs associated with the Glybera global registry and Phase IV study during the three months ended June 30, 2017, compared to \$0.9 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 \$0.3 million income from payments received from European authorities to subsidize our research and development efforts in the Netherlands in the three months ended June 30, 2017, compared to \$0.5 million for the same period in 2016.

We recognized other expense of \$1.7 million related to contractual commitments in relation to terminating the marketing of our Glybera program, as well as our collaborations with Chiesi in the three months ended June 30, 2017. We did not recognize any such expenses in the same period in 2016.

In the three months ended June 30, 2017, we accrued \$0.9 million as a result of the contract termination related to vacated facilities at our Amsterdam site. 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 June 30, 2017, and 2016 were as follows:

	Three months ended June 30,		
	2017	2016	2017 vs 2016
	in thousands		
Interest income	\$ 12	\$ 15	\$ (3)
Interest expense Hercules long-term debt	(502)	(549)	47
Foreign currency gains / (losses)	(1,071)	920	(1,991)
Other non-operating income	—	330	(330)
Total other non-operating income / (expense), net	\$ (1,561)	\$ 716	\$ (2,277)

We recognized a net foreign currency loss related to our borrowings from Hercules and our cash and cash equivalents of \$1.1 million during the three months ended June 30, 2017, compared to a net gain of \$0.9 million during the same period in 2016.

In the three months ended June 30, 2017, we recognized no result related to fair value changes of warrants compared to a gain of \$0.3 million for the same period in 2016.

Comparison of the six months ended June 30, 2017, and 2016

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

	Six months ended June 30,		
	2017	2016	2017 vs 2016
	in thousands		
Total revenues	\$ 8,263	\$ 8,746	\$ (483)
Operating expenses:			
Research and development expenses	(33,860)	(35,927)	2,067
Selling, general and administrative expenses	(11,768)	(15,132)	3,364
Total operating expenses	(45,628)	(51,059)	5,431
Other income	582	920	(338)
Other expense	(2,640)	—	(2,640)
Loss from operations	(39,423)	(41,393)	1,970
Other non-operating items, net	(2,118)	(1,762)	(356)
Loss before income tax benefit / (expense)	(41,541)	(43,155)	1,614
Income tax benefit / (expense)	—	(224)	224
Net loss	\$ (41,541)	\$ (43,379)	\$ 1,838

Revenue

Our revenue for the six months ended June 30, 2017, and 2016 was as follows:

	Six months ended June 30,		
	2017	2016	2017 vs 2016
	in thousands		
License revenue	\$ 1,944	\$ 2,475	\$ (531)
Collaboration revenue Chiesi	4,638	2,984	1,654
Collaboration revenue BMS	1,681	3,287	(1,606)
Total revenues	\$ 8,263	\$ 8,746	\$ (483)

In association with the upfront payments and target designation fees received from BMS in the second and third quarters of 2015, we recognized \$1.9 million in license revenue during the six months ended June 30, 2017, and 2016. In association with the upfront fees received from Chiesi in 2013, we recognized \$0.0 million license revenue during the six months ended June 30, 2017, compared to \$0.5 million during the same period in 2016. We recognized our license revenue for the six months ended June 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 will be required to repay in accordance with the termination of the Glybera Termination Agreement.

Collaboration revenue generated from research activities associated with our BMS-partnered S100A1 heart failure program during the six months ended June 30, 2017, was \$1.7 million, compared to \$3.3 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 activities related to the production of preclinical material during the same period in 2016.

Collaboration revenue generated from our co-development of hemophila B with Chiesi for the six months ended June 30, 2017, was \$4.6 million, compared to \$3.0 million for the same period in 2016.

Research and development expenses

Research and development expenses for the six months ended June 30, 2017, were \$33.9 million compared to \$35.9 million for the same period in 2016.

- We incurred \$18.4 million in personnel, share-based compensation expenses and consulting cost in the six months ended June 30, 2017, compared to \$18.8 million during the six months ended June 30, 2016. The decrease was a combination of \$0.9 million one-off expenses related to termination benefits and cost reductions resulting from our restructuring initiated in November 2016;
- We incurred \$7.1 million in external services and cost related to the development of our product candidates in the six months ended June 30, 2017, compared to \$9.2 million for the same period in 2016, as we incurred lower costs in relation to the manufacturing of drug substance to supply our programs;
- We incurred \$7.7 million operating expenses and depreciation expenses related to our rented facilities in the six months ended June 30, 2017, compared to \$6.7 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;
- We incurred no share-based compensation expenses related to our collaboration with 4D Molecular Therapeutics in the six months ended June 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 six months ended June 30, 2017, were \$11.8 million compared to \$15.1 million for the same period in 2016.

- Our expenses related to employees, contractors and consultants in the six months ended June 30, 2017, were \$4.2 million compared to \$4.6 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 same period in 2016;
- We incurred \$2.1 million of share-based compensation expenses in the six months ended June 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;
- We incurred \$2.8 million of professional fees in the six months ended June 30, 2017, compared to \$3.0 million for the same period in 2016. We regularly incur accounting, audit and legal fees associated with operating as a public company, including the cost of our conversion from IFRS to U.S. GAAP which we initiated during the second quarter 2016;
- We incurred legal and settlement costs of \$1.9 million in connection with our arbitration proceeding with Extera during the six months ended June 30, 2016. No such costs were incurred during the six months ended June 30, 2017; and
- We incurred \$0.3 million of costs associated with the Glybera global registry and Phase IV study during the six months ended June 30, 2017, compared to \$1.8 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 \$0.6 million income from payments received from European authorities to subsidize our research and development efforts in the Netherlands in the six months ended June 30, 2017, compared to \$0.9 million for the same period in 2016.

We recognized other expense of \$1.7 million related to contractual commitments in relation to terminating the marketing our Glybera program, as well as our collaborations with Chiesi in the six months ended June 30, 2017. We did not recognize any such expenses in the same period in 2016.

We accrued \$0.9 million of contract termination cost related to vacated facilities at our Amsterdam site in the six months ended June 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 six months ended June 30, 2017, and 2016 were as follows:

	Six months ended June 30,		
	2017	2016	2017 vs 2016
	in thousands		
Interest income	\$ 23	\$ 37	\$ (14)
Interest expense Hercules borrowing	(1,006)	(1,178)	172
Foreign currency losses	(1,164)	(1,268)	104
Other non-operating income	29	647	(618)
Total other non-operating income / (expense), net	\$ (2,118)	\$ (1,762)	\$ (356)

We recognized a net foreign currency loss related to our borrowings from Hercules and our cash and cash equivalents of \$1.2 million during the six months ended June 30, 2017, compared to a net loss of \$1.3 million during the same period in 2016.

In the six months ended June 30, 2017, we recognized no gain or loss related to fair value changes of warrants compared to a gain of \$0.6 million for the same period in 2016.

Financial Position, Liquidity and Capital Resources

As of June 30, 2017, we had cash and cash equivalents of \$104.1 million. We currently expect that our cash and cash equivalents will be sufficient to fund operations into 2019. The table below summarizes our consolidated cash flow data for the six months ended June 30, 2017, and 2016.

	Six months ended June 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	(30,803)	(38,469)
Net cash used in investing activities	(3,408)	(4,170)
Net cash generated from financing activities	939	2,097
Foreign exchange impact	4,863	2,848
Cash and cash equivalents at the end of the period	\$ 104,087	\$ 183,932

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 \$21.3 million and \$41.5 million, during the three and six months ended June 30, 2017, respectively, compared to a loss of \$21.1 million and \$43.4 million, during the respective periods in 2016. As of June 30, 2017, we had an accumulated deficit of \$437.6 million.

Sources of liquidity

From our first institutional venture capital financing in 2006 through June 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 extent 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.

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 \$30.8 million for the six months ended June 30, 2017, a reduction of \$7.7 million compared to the \$38.5 million of cash used in the same period in 2016.

The reduction is primarily due to a \$2.8 million decline in our net working capital during the six months ended June 30, 2017. In addition, we collected \$1.1 million in lease incentive payments related to our new Amsterdam facility and reduced our operating expenses during the six months ended June 30, 2017.

Net cash used in investing activities

In the six months ended June 30, 2017, we used \$3.4 million in our investing activities compared to \$4.2 million for the same period in 2016.

	Six months ended June 30,	
	2017	2016
	in thousands	
Build out of Lexington site	\$ (477)	\$ (1,179)
Build out of Amsterdam sites	(2,931)	(2,374)
Restricted cash	—	(617)
Total investments	\$ (3,408)	\$ (4,170)

In the six months ended June 30, 2017, we invested \$2.9 million in our new facility in Amsterdam and \$2.4 million in the same period 2016.

Net cash generated from financing activities

During the six months ended June 30, 2017, we received \$0.9 million from the exercise of options to purchase common 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 June 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 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, for 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 2017 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 hiring additional 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 June 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 \$4.0 million interest payments)	\$	\$ 5,824	\$ 9,098	\$ 9,080	\$ —	\$ 24,002
Operating lease obligations	—	2,772	3,774	11,643	21,902	40,091
Contingent consideration (nominal amount)	16,565	—	—	—	—	16,565
Total	\$ 16,565	\$ 8,596	\$ 12,872	\$ 20,723	\$ 21,902	\$ 80,658

Due to uncertainty of the timing of achieving certain contractual milestones, the contingent consideration of \$16.6 million related to our acquisition of InoCard (later renamed uniQure GmbH) is considered to have an undefined contractual maturity. As of June 30, 2017, we expect the milestone obligations will become payable between 2018 and 2021. When due, the obligations can be settled either in cash or in a variable number of our shares. As of June 30, 2017, we recorded this obligation at its fair value of \$2.4 million. [see above regarding the financial impact of the July 2017/August 2017 Amendment of the Sale and Purchase Agreement]

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 June 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 six months ended June 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 interim 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 June 30, 2017. Based on such evaluation, our CEO has concluded that as of June 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 six months ended June 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 common 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, filed with the SEC on March 15, 2017, before deciding to invest in our common 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 a sufficient number of 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. In particular, 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 in light of 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 as a result of many factors, including changes in regulatory policy during the period of product development. Failure to confirm favorable results from earlier trials by demonstrating the safety and effectiveness of our products in late-stage clinical trials with larger patient populations could have a material adverse effect on our business that would cause our share price to decline.

Fast track product, breakthrough therapy, priority review, or Regenerative Advanced Therapy (“RAT”) 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, RAT 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 RAT 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, as long as 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, which 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, RAT, 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, RAT, 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, with regard to 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, RAT, or a breakthrough therapy or, for priority review products, decide that the time 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 a number of more established companies are also pursuing strategies to license or acquire product candidates or technologies that we may consider attractive. These established companies may have a competitive advantage over us due to their size, cash resources and greater clinical development and commercialization capabilities. In addition, companies that perceive us to be competitors may be unwilling to license rights to us. Furthermore, we may be unable to identify suitable product candidates or technologies within our areas of focus. If we are unable to successfully obtain rights to suitable product candidates or technologies, our business, financial condition and prospects could suffer.

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 June 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

Delays in certifying our U.S. manufacturing facility could negatively impact our development and commercialization plans and thereby limit our revenues and growth. *

We have commenced consolidating all manufacturing at our facility in Lexington, Massachusetts. Certification of this facility is required to produce material used in clinical studies in the European Union as well as for commercial use. If we are unable to obtain the appropriate certification of our Lexington manufacturing facility, we may not be able to manufacture sufficient quantities of our product candidates, which would limit our commercialization and development activities and our opportunities for growth. Cost overruns associated with this facility could also require us to raise additional funds from external sources, which may be unavailable on favorable terms or at all.

Our manufacturing facility 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. *

We have decommissioned our GMP certified facility in Amsterdam and we are transferring all GMP manufacturing activities to our facility in Lexington, Massachusetts. 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. We may encounter problems in completing our technology transfer or in achieving adequate or clinical-grade materials that meet EMA, FDA or other applicable standards or specifications with consistent and acceptable production yields and costs.

A number of 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, the Netherlands and Germany 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

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.

Regulators will require us to complete studies demonstrating comparability between product manufactured at our Amsterdam facility, which was used in our Phase I/II study in hemophilia B, and product manufactured at our Lexington, Massachusetts facility, which will be used for our hemophilia B pivotal study and ultimately commercial use. Those studies and their results could substantially delay the development and approval of our hemophilia B product candidate.

The FDA maintains strict requirements governing the manufacturing process for biologics. When a manufacturer seeks to modify or change that process, or begin manufacturing at a new facility, the FDA typically requires the applicant to conduct non-clinical and, depending on the magnitude of the changes, potentially clinical comparability studies that evaluate the potential differences in the product resulting from the change in the manufacturing process or facility. In connection with any application for marketing approval in the United States, we will be required to conduct comparability studies assessing product manufactured at our facility in Amsterdam with product to be manufactured at our new facility in Lexington, Massachusetts.

Delays in designing and completing a comparability study to the satisfaction of the FDA could delay or preclude our development and commercialization plans and, thereby, increase the risk and time to achieve regulatory approval. For example, we may attempt to show comparability of the product manufactured at our Amsterdam and Lexington facilities through the use of non-clinical data, such as potency assays and animal studies. In the event that the FDA does not accept such non-clinical comparability data, we may need to conduct additional studies involving dosing of animals or patients. These potential studies may result in a delay of the approval or launch of product in the United States.

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 in order for our product candidates to gain regulatory approval or change the requirements for tracking, handling and distribution of the products which may be associated with increased costs. In addition, divergent scientific opinions among the various bodies involved in the review process may result in delays, require additional resources and ultimately result in rejection. These regulatory agencies, committees and advisory groups and the new regulations and guidelines they promulgate may

lengthen the regulatory review process, require us to perform additional studies, increase our development costs, lead to changes in regulatory positions and interpretations, delay or prevent approval and commercialization of our product candidates or lead to significant post-approval limitations or restrictions. Delay or failure to obtain, or unexpected costs in obtaining, the regulatory approval necessary to bring a potential product to market could decrease our ability to generate sufficient product revenues to maintain our business.

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

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

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

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 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 a number of factors, including:

- successful completion of preclinical studies and clinical trials;
- receipt and maintenance of marketing approvals from applicable regulatory authorities;
- our ability to timely manufacture sufficient quantities according to required quality specifications;
- obtaining and maintaining patent and trade secret protection and non-patent, orphan drug exclusivity for our product candidates;
- obtaining and maintaining regulatory approval for our manufacturing facility in Lexington, Massachusetts;

- launch and commercialization of our products, if and when approved, whether alone or in collaboration with others;
- identifying and engaging effective distributors or resellers on acceptable terms in jurisdictions where we plan to utilize third parties for the marketing and sales of our product candidates;
- acceptance of our products, if and when 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 a number of factors, including the diagnosis and treatment criteria included in the final label, if approved for sale in specified indications, acceptance by the medical community, patient consent, patient access and product pricing and reimbursement. 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-060 gene therapy candidate for hemophilia B patients incorporates an AAV5 capsid. In our Phase I/II clinical study, we screened patients for preexisting anti-AAV5 antibodies in order 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 those 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-060 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 a number of 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.

In order 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.

In connection with our 2014 acquisition of the InoCard business (later renamed uniQure GmbH), we undertook certain obligations regarding the development of the acquired program pursuant to a plan to be agreed between us and the sellers. The acquisition agreement provides that, in the case of an unremedied breach by us of these development obligations, the sellers could be entitled, in defined circumstances, to repurchase the InoCard business from us. If we were to breach such development obligations and were not successful in remedying such breach, the sellers might seek to exercise this repurchase right or to claim other financial penalties. Although we are diligently pursuing the development of the acquired program through our collaboration with BMS, and believe that we have not breached and will not breach such development obligations, any claim of breach could result in distraction of management and staff attention. In the event that the sellers were successful in pursuing a claim of breach by us of such obligations, our financial position and our efforts to develop S100A1 together with our collaboration partner BMS could be materially adversely affected.

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 from 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 of 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 behind the actual discoveries, and patent applications in the United States and other jurisdictions are typically not published until 18 months after their priority date, or in some cases at all. Therefore, we cannot know with certainty whether we were the first to make the inventions or that we were the first to file for patent protection of the inventions claimed in our owned or licensed patents or pending patent applications. As a result, the issuance, scope, validity, enforceability and commercial value of our patent rights are highly uncertain. Our pending and future patent applications may not result in patents being issued that protect our technology or products, in whole or in part, or which effectively prevent others from commercializing competitive technologies and products. Changes in either the patent laws or interpretation of the patent laws in the European Union, the United States or other countries may diminish the value of our patents or narrow the scope of our patent protection.

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

Competitors may infringe our owned or licensed patents or other intellectual property. To counter infringement or unauthorized use, we may be required to file infringement claims, which can be expensive and time consuming. An

adverse result in any litigation proceeding could put one or more of our patents at risk of being invalidated, maintained in more narrowly amended form or interpreted narrowly.

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

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

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

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

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

In addition, these agreements typically restrict the ability of our collaborators, advisors and consultants to publish data potentially relating to our trade secrets. Our academic collaborators typically have rights to publish data, provided that we are notified in advance and may delay publication for a specified time in order to secure our intellectual property rights arising from the collaboration. In other cases, publication rights are controlled exclusively by us, although in some cases we may share these rights with other parties. We also conduct joint research and development programs that may require us to share trade secrets under the terms of our research and development partnerships or similar agreements.

Some courts inside and outside the United States are less willing or unwilling to protect trade secrets. If any of our trade secrets were to be lawfully obtained or independently developed by a competitor, we would have no right to prevent them, or those to whom they communicate it, from using that technology or information to compete with us.

Risks Related to Pricing and Reimbursement

We face uncertainty related to insurance coverage of, and pricing and reimbursement for 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 in light of 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. As a result 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 in order to increase the likelihood of obtaining reimbursement approvals. In the event that countries impose prices, which are not sufficient to allow us or our collaborators to generate a profit, we or our collaborators may refuse to launch the product in such countries or withdraw the product from the market. If pricing is set at unsatisfactory levels, or if the price decreases, our business could be harmed, possibly materially. If we fail to obtain and sustain an adequate level of coverage and reimbursement for our products by third party 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 particular 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, in light of 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 \$41.5 million in the six month period ended June 30, 2017, \$73.4 million in full year 2016 and \$82.1 million in 2015. As of June 30, 2017, we had an accumulated deficit of \$437.6 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 of 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 our gene therapy candidate in hemophilia B;
- advance the preclinical development and initiate a clinical study for 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 in order 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 of our assets as collateral. If we raise additional capital through the sale of equity or convertible debt securities, our shareholders' ownership interest will be diluted, and the terms of these securities may include liquidation or other preferences that adversely affect the rights of holders of our common 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.

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

As of June 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 2017 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 of our assets.

We may be required to sublease excess space in our Amsterdam site.

In March 2016, we entered into a lease for a new approximately 100,000 square feet facility in Amsterdam, and we amended this agreement in June 2016 in order to lease an additional 11,000 square feet. The lease for this facility terminates in 2032. Following our restructuring announced in November 2016, we do not expect to utilize a significant portion of our new Amsterdam facility. We are contractually required to incur significant costs in relation to areas not utilized by us over the full contractual term. While we will seek to sublease any excess space, we may not be able to do so at commercially attractive terms.

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 in order to comply with current or future environmental, health and safety laws and regulations. These current or future laws and regulations may impair our research, development or production efforts. Our failure to comply with these laws and regulations also may result in substantial fines, penalties or other sanctions.

Product liability lawsuits 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 of time because of the limited number of individuals in our industry with the breadth and depth of skills and experience required to successfully develop gene therapy products. Competition to hire from this limited pool is intense, and we may be unable to hire, train, retain or motivate these key personnel on acceptable terms.

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

We have initiated a significant restructuring of our operations that will include a sizable reduction in headcount in our Amsterdam facility and expansion of personnel in our Lexington facility, and as a result, we may encounter challenges managing the associated organizational change.*

In November 2016, we announced plans to restructure and refocus our business, which will result in the elimination of approximately 50 to 55 net positions, as well as the transition of certain operations to our Lexington, Massachusetts facility. We will incur expenses related to the reduction in staff as well as the retention of key personnel. We may also experience operational disruptions as we implement our new organizational structure and transfer certain functions to Lexington. This process may distract the attention of management and staff and may cause disruption in our operations.

At the same time, we continue to expand the scope of certain of our operations in the United States, particularly in the areas of clinical operations, medical and regulatory affairs and product development. Due to our limited financial resources and the limited experience of our management team in implementing significant organizational change, we may not be able to effectively manage this process.

Risks Related to Our Common Shares

The price of our common 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 common shares on the NASDAQ Global Select Market on February 4, 2014 through August 3, 2017, the sale price of our common shares ranged from a high of \$36.38 to a low of \$4.91. The closing price on August 3, 2017, was \$8.07 per common 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 common 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 common shares may not be sustained.

Although our common 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 common 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 common 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 common shares, in the aggregate, beneficially own approximately 31.4% of our issued shares as at June 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 of our assets.

Provisions of our articles of association or Dutch corporate law might deter acquisition bids for us that might be considered favorable and prevent or frustrate any attempt to replace 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 common shares and any returns on an investment in our common shares will likely depend entirely upon any future appreciation in the price of our common shares.

We are an "emerging growth company," and the reduced disclosure requirements applicable to emerging growth companies may make our common 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 common shares less attractive as a result of our reliance on these exemptions, trading market for our common 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 common 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 common 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

For U.S. tax purposes, we qualify as a passive foreign investment company as of December 31, 2016, 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 for the production of passive income. Passive income for this purpose generally includes dividends, interest, royalties, rents and gains from commodities and securities transactions. Our status in any taxable year will depend on our assets and activities in each year, and because this is a factual determination made annually after the end of each taxable year, there can be no assurance that we will 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 common shares, which is likely to fluctuate, and may fluctuate considerably given that market prices of biotechnology companies have been especially volatile. As we are a PFIC for the taxable year 2016, certain adverse U.S. federal income tax consequences, including reporting obligations, could apply to a U.S. holder who held our common shares during 2016.

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 managing directors or supervisory directors in an original action based solely upon the federal securities laws of the United States brought in a court of competent jurisdiction in the Netherlands.

The United States and the Netherlands currently do not have a treaty providing for the reciprocal recognition and enforcement of judgments, other than arbitration awards, in civil and commercial matters. Consequently, a final judgment for payment given by a court in the United States, whether or not predicated solely upon U.S. securities laws, would not automatically be recognized or enforceable in the Netherlands. In order to obtain a judgment which is enforceable in the Netherlands, the party in whose favor a final and conclusive judgment of the U.S. court has been rendered will be required to file its claim with a court of competent jurisdiction in the Netherlands. Such party may submit to the Dutch court the final judgment rendered by the U.S. court. If and to the extent that the Dutch court finds that the jurisdiction of the U.S. court has been based on grounds which are internationally acceptable and that proper legal procedures have been observed, the Dutch court will, in principle, give binding effect to the judgment of the U.S. court, unless such judgment contravenes principles of public policy of the Netherlands. Dutch courts may deny the recognition and enforcement of punitive damages

or other awards. Moreover, a Dutch court may reduce the amount of damages granted by a U.S. court and recognize damages only to the extent that they are necessary to compensate actual losses or damages. Enforcement and recognition of judgments of U.S. courts in the Netherlands are solely governed by the provisions of the Dutch Civil Procedure Code.

Therefore U.S. shareholders may not be able to enforce against us or our 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

None.

Item 5. Other Information

None.

Item 6. Exhibits

See the Exhibit Index following 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.

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.

UNIQUE, N.V.

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

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 July 27, 2017 between uniQure biopharma B.V. and Chiesi Farmaceutici S.p.A.

10.2* Employment Agreement dated August 4, 2017 between uniQure biopharma B.V. and Sander van Deventer

10.3* Employment Agreement dated July 10, 2017 between uniQure, Inc. and Scott McMillan

10.4* Employment Agreement dated July 15, 2017 between uniQure biopharma B.V. and Christian Klemt

31.1* Rule 13a-14(a)/15d-14(a) Certification of Chief Executive Officer

31.2* Rule 13a-14(a)/15d-14(a) Certification of Chief Executive Officer

32.1* Section 1350 Certification (furnished herewith)

101* The following financial information from our Quarterly Report on Form 10-Q for the period ended June 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.

uniQure biopharma B.V.
Paasheuvelweg 25a
1105BP Amsterdam
The Netherlands

26 July 2017

VIA E-MAIL AND BY OVERNIGHT COURIER

Chiesi Farmaceutici S.p.A.
Via Palermo, 26/A
43122 Parma
Italy
Attention: CEO
Copy to: Corporate Development, Head and General Counsel

Gentlemen:

1. Agreement

I am writing to confirm the terms and conditions on which uniQure biopharma B.V. (“**uniQure**”) and Chiesi Farmaceutici S.p.A. (“**Chiesi**”) (together, the “**Parties**”, each a “**Party**”) have agreed to terminate the agreement (as better defined below) between them relating to a certain gene therapy product including an AAV5 Vector containing a certain human Factor IX gene (or part thereof) construct for the treatment of hemophilia B in humans (the “**Product**”). As part of that agreement, Chiesi has agreed that it will undertake a smooth and orderly transfer, pursuant to the terms of paragraph 5.1 below, of all activities relating to the development or other exploitation of the Product currently being undertaken by Chiesi, to uniQure (the “**Transfer**”) and that the termination agreement between the Parties relating to Glybera dated 19 April 2017 (the “**Glybera Termination Agreement**”) will be amended as provided in this letter agreement.

EMA: 1133572-1

2. Termination

- 2.1 The Parties have mutually agreed to terminate the Co-Development and License Agreement dated 29 April 2013 between uniQure and Chiesi (the “**Agreement**”). Subject to paragraph 2.4 and 5.1 of this letter agreement, each Party acknowledges and agrees that the Agreement shall terminate in their entirety and shall be of no further force and effect (the “**Termination**”), on ____ July 2017 (the “**Termination Date**”).
- 2.2 Chiesi hereby represents and warrants to uniQure that, to its knowledge and as of the Termination Date, no Chiesi Technology is incorporated into the Product, or is required for the development, manufacture, commercialisation or other exploitation of the Product in or outside the Territory (the “**IP Warranty**”). Chiesi hereby absolutely, unconditionally and irrevocably, covenants and agrees to uniQure, its Affiliates and its and their licensees, assigns and successors in interest (each a “**uniQure Party**”) that Chiesi will not and it will procure that its Affiliates and licensees will not, directly or indirectly, assert any Chiesi Technology or bring, initiate, continue, maintain or issue any claim, cause of action or proceeding (at law, in equity, in any regulatory proceeding or otherwise) against any uniQure Party, whether as a claim, cross-claim, counterclaim or otherwise, in each case with respect to the development, manufacture, commercialisation or any other exploitation of the Product by any uniQure Party in or outside the Territory, except to the extent uniQure breaches Section 6.2 of this letter agreement (each such claim, cause of action or proceeding, a “**Released Claim**”). If Chiesi breaches this Section 2.2 by bringing, initiating, continuing, maintaining or issuing any Released Claim, then Chiesi shall indemnify such uniQure Party against all Losses it incurred in defending such Released Claim, except to the extent uniQure breaches Section 6.2 of this letter agreement (the “**IP Indemnity**”). Each uniQure Party is a beneficiary to the provisions of this Section 2.2 and is entitled to the rights and benefits hereunder and may enforce such provisions as if it were a party under this letter agreement, except for uniQure’s and its Affiliates’ licensees which may only enforce such provisions through uniQure.
- 2.3 For the avoidance of doubt and except to the extent uniQure breaches Section 6.2 of this letter agreement, if there is a breach of the IP Warranty: (a) uniQure shall not have a right to recover any Losses incurred as a result of such breach under the IP Warranty, to the extent any uniQure Party has been fully compensated for such Losses under the IP Indemnity, and (b) no uniQure Party shall have a right to be compensated for any Losses incurred as a result of such breach under the IP Indemnity, to the extent uniQure has fully recovered such Losses under the IP Warranty.
- 2.4 The following terms of the Agreement shall survive the termination of the Agreement on the Termination Date: Article 1, Section 6.1 (subject to the terms of Section 12.5(i)), Section 7.2, Section 7.3, Section 9.1, Section 10.1, Section 10.2, Section 10.7, Section 11.5, Sections 12.5(a), 12.5(h), 12.5(i); Article XIII, Article XIV, and Article XV. For the avoidance of doubt, all other terms, provisions, rights and obligations under the Agreement are, and shall be deemed to be, terminated and of no further force or effect as of the Termination Date.

3. Payments

- 3.1 As a full and final settlement of all sums owed and payable by Chiesi to uniQure under Article VIII of the Agreement (excluding the amounts due in invoice number 2017.3007 dated May 1, 2017 for EUR ** (** Euros) invoice number 2017.3004-b dated July 25, 2017 for EUR ** (** Euros) provided to Chiesi by uniQure, which notwithstanding paragraph 6.2 of this letter agreement, Chiesi will pay in accordance with the Agreement) Chiesi shall owe EUR ** (** Euros) to uniQure to be satisfied in accordance with paragraph 3.2 of this letter agreement.
-

- 3.2 The parties agree that the Base Amount, the Patient 1 Amount and the Patient 2 Amount (each as defined in the Glybera Termination Agreement) shall be due as of the Termination Date and that Chiesi shall be entitled to withhold EUR ** (** Euros) from the amount otherwise payable pursuant to paragraph 3.1 of this letter agreement. For the avoidance of doubt, as a result of such set off, Chiesi hereby waives and releases uniQure in full from any further payment obligations of uniQure with respect to the Base Amount, the Patient 1 Amount and Patient 2 Amount. Chiesi shall pay the balance of the amount due to uniQure (EUR **), (** Euros) without any deduction, to the bank account specified by uniQure within 15 (fifteen) days of the Termination Date and after having received from uniQure one (1) final invoice for the remaining period till the Termination Date pursuant to the form attached as Exhibit 3 hereto.
- 3.3 All amounts due under this Section 3 are exclusive of any Value Added Tax (which, if applicable, shall be payable by Chiesi in addition to such amounts due upon receipt of a valid Value Added Tax invoice). All amounts due under this Section 3 shall be made without any deduction or withholding for or on account of any tax unless such deduction or withholding is required by Applicable Laws, in which case the sum payable by Chiesi shall be increased to the extent necessary to ensure that, after the making of such deduction or withholding, uniQure receives and retains (free from any liability in respect of any such deduction or withholding) a net sum equal to the sum it would have received had no such deduction or withholding been made or required to be made. If uniQure subsequently receives a credit for such deduction or withholding it shall forthwith pay the amount of such credit to Chiesi. No credit shall have been received by uniQure unless it shall have relieved uniQure of a present obligation to pay tax.

4. **Glybera Termination Agreement**

- 4.1 Each Party acknowledges and agrees that, with effect from the Termination Date, the Glybera Termination Agreement is amended as follows:
- (a) subparagraph 3 of the third paragraph of Section 3 of the Glybera Termination Agreement shall be deleted in its entirety, such that the Additional Patient Amount (as defined in the Glybera Termination Agreement) shall no longer apply and
 - (b) the phrase “and Additional Patient Amount” shall be deleted in its entirety from subparagraph 5 of the fourth paragraph of Section 3 of the Glybera Termination Agreement.

5. **Handover**

- 5.1
- (a) The following provisions shall apply to the Termination, it being understood and agreed that all information, documents, materials, records, including Product Data and Product Information, to be provided or transferred by Chiesi hereunder will be provided or transferred on a “as is” basis and without giving any warranty, express or implied, on the status, merchantability, fitness for a particular purpose and non-infringement thereof:
 - (i) Exhibit 1 attached hereto contains a fair and reasonably accurate description of the status of Development Program activities conducted by Chiesi before the Termination Date and a list of documents generated thereunder (the “**Documents List**”);
 - (ii) Chiesi hereby assigns to uniQure the entire right, title and interest in and to, any Product Data in Chiesi’s or its Affiliates’ or Third Party contractors’ possession or Control;
-

- (iii) within thirty (30) days after the Termination Date, Chiesi shall assign, on a “as is” basis and without giving any warranty, express or implied, on the status, validity and enforceability thereof, the agreement titled “**ACCORDO UNILATERALE DI CONFIDENZIALITÀ**” entered into with Professor Carlo Ferrari on January 7, 2015 to uniQure (the “**Third Party Agreement**”), a copy of which is attached hereto as Exhibit 2; and
 - (iv) each Party acknowledges and agrees that, with effect from the Termination Date, for the purposes of Article X of the Agreement:
 - (1) all Know-How with respect to the Product or the Development Program (including the Product Data, and other Know-How contained in the notes, records, minutes, documents, reports, records, dossiers, correspondence or material (as applicable) described in Sections 5.1(b), (c) or (d)), and the terms and conditions of the Third Party Agreement) (together, the “**Product Information**”), shall be deemed to be the Confidential Information of uniQure (and for the avoidance of doubt, shall not be the Confidential Information of Chiesi), and the terms and conditions of this letter agreement shall be the Confidential Information of each Party; and
 - (2) Sections 10.1 (a), (b) and (d) of the Agreement shall not apply with respect to the Product Information generated by Chiesi or the terms and conditions of this letter agreement;
 - (b) within thirty (30) days after the Termination Date, Chiesi shall destroy and certify in writing to uniQure that it has destroyed all materials and records in its possession or Control containing Confidential Information of uniQure (including Product Information), except for a single copy of such Confidential Information that may be retained confidentially for legal purposes only; it is however understood and agreed that in the event Chiesi discovers, within one (1) year after the Termination Date, any other materials and/or records containing Confidential Information of uniQure (including Product Information), the provisions of this sub-paragraph (b) shall seamlessly apply.
 - (c) within thirty (30) days after the Termination Date, Chiesi shall provide uniQure full access (with rights to download all documents) to the Sharepoint or other network drive containing all notes, records, minutes and documents with respect to all JSC meetings, JDC meetings and JCC meetings, copies of Product Data not previously provided to uniQure and copies of the documents referenced to in the Documents List and shall maintain such access for a period of one (1) month after such full access is granted to uniQure;
 - (d) within thirty (30) days after the Termination Date, Chiesi shall transfer to uniQure copies of any market research reports under Chiesi’s or its Affiliates’ or Third Party contractors’ possession or Control with respect to the Products, and transfer to uniQure all copies of the notes, records, dossiers and other documents prepared for or with respect to any meeting with the Regulatory Authorities (including the EMA and any Health Technology Assessments) with respect to the Product under Chiesi’s or its Affiliates’ possession or Control to the extent not already provided to uniQure pursuant to paragraph (c) above;
 - (e) from the Termination Date, as between uniQure and Chiesi, uniQure shall have the sole right to prepare, obtain and maintain Regulatory Approvals, and to conduct communications with the Regulatory Authorities, in respect to the development, manufacture, commercialisation and other exploitation of the Product (such activities, the “**Regulatory Activities**”). Chiesi shall promptly provide (and in any event, no later than five (5) Business Days of receipt) copies of
-

any written or electronic correspondence relating to the development, manufacture, commercialisation and other exploitation of the Product which Chiesi or its Affiliates received from the Regulatory Authorities;

- (f) Chiesi shall ensure that those of Chiesi's employees whose decisions or inputs are reasonably necessary for the activities necessary to enable the Transfer are available to uniQure, during one teleconference to be held within thirty (30) days after the Termination Date, to respond to any of uniQure's queries on any matter relating to the Transfer, provided that uniQure has sent to Chiesi any such written questions upon the Termination Date; and
- (g) Chiesi shall within thirty (30) days after the Termination Date execute and deliver, or procure any necessary third party shall within thirty (30) days after the Termination Date execute and deliver, any documents as may be necessary to enable the Transfer.

6. Release of the Parties

- 6.1 Release of the uniQure Released Parties. Effective as of the Termination Date, Chiesi, on behalf of itself and each of its agents, principals, officers, directors, employees, stockholders, partners, parents, subsidiaries, affiliates, predecessors, successors, representatives, and assigns ("**Chiesi Affiliates**"), fully, finally and forever releases relinquishes and discharges uniQure and any acquirer or assignee of uniQure's assets and their respective past, present or future officers, directors, shareholders, joint venturers, affiliates, members, partners, partnerships, principals, parent companies, subsidiaries, representatives, employees, servants, and agents, in their capacities as such (collectively, the "**uniQure Released Parties**"), of and from any and all charges, complaints, claims, liabilities, obligations, promises, agreements, controversies, damages or causes of action, suits, rights, demands, costs, losses, debts and expenses (including attorneys' fees and costs incurred) of any nature whatsoever, in law or in equity, whether known or unknown, anticipated or unanticipated, and whether accrued or hereafter to accrue that they now have, may have, or could have from the beginning of time to the Termination Date that in any way arises out of, are connected with, or that are in any way related to, the Agreement, excluding only claims for breach of this letter agreement and the provisions, rights and obligations of the parties that expressly survive the Termination Date as set forth in this letter agreement.
 - 6.2 Release of the Chiesi Released Parties. Effective as of the Termination Date, uniQure, on behalf of itself and each of its agents, principals, officers, directors, employees, stockholders, partners, parents, subsidiaries, affiliates, predecessors, successors, representatives, and assigns ("**uniQure Affiliates**"), fully, finally and forever releases relinquishes and discharges Chiesi and any acquirer or assignee of Chiesi's assets and their respective past, present or future officers, directors, shareholders, joint venturers, affiliates, members, partners, partnerships, principals, parent companies, subsidiaries, representatives, employees, servants, and agents, in their capacities as such (collectively, the "**Chiesi Released Parties**"), of and from any and all charges, complaints, claims, liabilities, obligations, promises, agreements, controversies, damages or causes of action, suits, rights, demands, costs, losses, debts and expenses (including attorneys' fees and costs incurred) of any nature whatsoever, in law or in equity, whether known or unknown, anticipated or unanticipated, and whether accrued or hereafter to accrue that they now have, may have, or could have from the beginning of time to the Termination Date that in any way arises out of, are connected with, or that are in any way related to, the Agreement, excluding only claims for breach of this letter agreement and the provisions, rights and obligations of the parties that expressly survive the Termination Date as set forth in this letter agreement.
-

7. Public Announcements

- 7.1 Subject to Sections 7.2 and 7.3 of this letter agreement, neither Party may issue any announcement, press release or make any such other public statement, in each case, with respect to or in connection with the Termination or this letter agreement, without consent of the other Party. The Parties shall consult together on the timing, contents and manner of release of any such announcement, press release or public statement.
- 7.2 The Parties agree to make an announcement with respect to the Termination, in the form set out in the Appendix, within two (2) Business Days of the Termination Date (the “**Announcement**”). Thereafter, each Party may, without consultation or consent from the other Party, make any public statement in response to questions from the press, research analysts, investors or those attending industry conferences, make internal announcements to employees and make disclosures in documents filed by uniQure with the SEC, so long as such statements, announcements and disclosures substantially reiterate the Announcement or the information within it, and are not inconsistent with the Announcement.
- 7.3 Where an announcement, press release or public statement is required by Applicable Laws, any Regulatory Authority or governmental authority (including the SEC), or by any court or other authority of competent jurisdiction, the Party required to make such announcement, press release or public statement shall promptly notify the other Party, it shall consult with the other Party about, and shall use its best reasonable efforts to provide the other Party time to comment on, such release or announcement in advance of such issuance, and the required Party will consider such comments in good faith.

8. Interpretation

- 8.1 Capitalised terms not otherwise defined in this letter agreement shall have the meaning set out in the Agreement.
- 8.2 Section 1.130 (a), (b), (c), (d), (e), (f), (g), (h) and (k) of the Agreement shall be incorporated into this letter agreement by reference, with the necessary changes made.

9. Execution as a Deed

- 9.1 Chiesi and uniQure agree that this letter agreement shall be a deed.

10. Other Provisions

- 10.1 Article XV of the Agreement shall be incorporated into this letter agreement by reference.

This document is hereby executed by each of the Parties as a deed and is delivered and takes effect on the date executed by Chiesi.

UNIQUE BIOPHARMA B.V.

Executed as a deed by uniQure Biopharma B.V., a company incorporated in the Netherlands, by the following persons, each being a person who, in accordance with the laws of the Netherlands, is acting under the authority of the company.

By: /s/ Matthew Kapusta
Name: Mr. Matthew Kapusta
Title: CEO

By: /s/ Christian Klemt
Name: Mr. Christian Klemt
Title: Global Controller
Date: 26/07/2017

Chiesi hereby acknowledges and agrees to any and all of the terms set out in this letter agreement and hereby executes and delivers this agreement as deed.

CHIESI FARMACEUTICI S.P.A.

Executed as a deed by Chiesi Farmaceutici S.p.A, a company incorporated in Italy, by the following persons, each being a person who, in accordance with the laws of Italy, is acting under the authority of the company.

By: /s/ Ugo Di Francesco

Name: Mr. Ugo Di Francesco

Title: CEO

By: /s/ Paolo Chiesi

Name: Mr. Paolo Chiesi

Title: Vice President

APPENDIX

Form of Press Release

uniQure Reacquires Development and Commercialization Rights for its Gene Therapy Candidate in Hemophilia B

~ Company now owns full global rights to late-stage program with clinical proof-of-concept ~

Lexington, MA and Amsterdam, the Netherlands, July __, 2017 — uniQure N.V. (NASDAQ: QURE), a leading gene therapy company advancing transformative therapies for patients with severe medical needs, today announced that it has entered into an agreement with Chiesi Group to reacquire the rights to co-develop and commercialize its hemophilia B gene therapy in Europe and other select territories and to terminate their co-development and license agreement.

“We are very pleased to reach an agreement with Chiesi to acquire back European and other territorial rights to our lead gene therapy program in hemophilia B,” stated Matthew Kapusta, Chief Executive Officer of uniQure. “By regaining unencumbered, global rights to a late-stage program that has demonstrated significant clinical benefit for patients with hemophilia B, we believe uniQure is better positioned to accelerate the global clinical development plan, maximize shareholder return on our pipeline and take advantage of new potential opportunities related to the program. We are grateful for the substantial investments that Chiesi has made in AMT-060, and we have been fortunate to have them as a collaboration partner over the years.”

“We have recently made significant progress in preparing for a late-stage clinical program in hemophilia B and will be providing several updates throughout the second half of this year,” added Mr. Kapusta.

“Chiesi’s decision was driven by recent changes in our strategic priorities,” stated Ugo Di Francesco, Chief Executive Officer of Chiesi. “We greatly appreciate the advances uniQure has made in the development of AMT-060 over the years and sincerely wish them the best as they advance this potentially exciting gene therapy to patients. We will continue to support the transition and expect it will be relatively quick and seamless.”

In 2013, uniQure and Chiesi entered into an agreement for the co-development and commercialization of a hemophilia B gene therapy in Europe and other select territories, including an equal sharing of all development related costs. Under the terms of the agreement announced today, uniQure will be responsible for all future development costs related to its hemophilia B program, including approximately \$3 million of expenses in 2017 that would have otherwise been shared with Chiesi. The Company does not expect the transaction will impact its previous cash guidance, and continues to anticipate cash on hand will be sufficient to fund operations into 2019.

As a result of the transaction, uniQure expects to recognize in the third quarter of 2017 the remaining deferred revenue of approximately \$14 million from non-refundable payments received from Chiesi in 2013.

About uniQure

uniQure is delivering on the promise of gene therapy – single treatments with potentially curative results. We are leveraging our modular and validated technology platform to rapidly advance a pipeline of proprietary and partnered gene therapies to treat patients with hemophilia, Huntington's disease and cardiovascular diseases. www.uniQure.com

About Chiesi Group

Based in Parma, Italy, Chiesi Farmaceutici is an international research-focused Healthcare Group, with over 80 years of experience in the pharmaceutical industry, present in 26 countries. Chiesi researches, develops and markets innovative drugs in the respiratory therapeutics, specialist medicine and rare disease areas. Its R&D organization is headquartered in Parma (Italy), and integrated with 6 other key R&D groups in France, the USA, the UK, Sweden and Denmark to advance Chiesi's pre-clinical, clinical and registration programmes. Chiesi employs nearly 5,000 people.

uniQure Forward-Looking Statements

This press release contains forward-looking statements. All statements other than statements of historical fact are forward-looking statements, which are often indicated by terms such as "anticipate," "believe," "could," "estimate," "expect," "goal," "intend," "look forward to", "may," "plan," "potential," "predict," "project," "should," "will," "would" and similar expressions. Forward-looking statements are based on management's beliefs and assumptions and on information available to management only as of the date of this press release. These forward-looking statements include, but are not limited to, statements regarding the future development of our hemophilia B program, the transition of development efforts from Chiesi and the risk of cessation, delay or lack of success of any of our ongoing or planned clinical studies and/or development of our product candidates. Our actual results could differ materially from those anticipated in these forward-looking statements for many reasons, including, without limitation, risks associated with corporate reorganizations and strategic shifts, collaboration arrangements, our and our collaborators' clinical development activities, regulatory oversight, product commercialization and intellectual property claims, as well as the risks, uncertainties and other factors described under the heading "Risk Factors" in uniQure's 2016 Annual Report on Form 10-K filed on March 15, 2017. Given these risks, uncertainties and other factors, you should not place undue reliance on these forward-looking statements, and we assume no obligation to update these forward-looking statements, even if new information becomes available in the future.

uniQure Contacts:

Maria E. CantorTom Malone

Direct: 339-970-7536Direct: 339-970-7558

Mobile: 617-680-9452Mobile: 339-223-8541

m.cantor@uniQure.com t.malone@uniQure.com

Eva M. Mulder

Direct: +31 20 240 6103

Mobile: +31 6 52 33 15 79

e.mulder@uniQure.com

**EMPLOYMENT AGREEMENT PURSUANT TO
SECTION 7:610 (et seq.) of the Dutch Civil Code
(DCC)**

August 4, 2017

Employment agreement between

- (1) **uniQure biopharma B.V.**, a company with limited liability (*besloten vennootschap met beperkte aansprakelijkheid*), with registered office at Amsterdam and principal place of business at Paasheuvelweg 25a, (1105 BP) Amsterdam (the **Employer**); and
 - (2) **Sander van Deventer** born on _____, residing at the address _____ (the **Employee**)
- each "a Party", collectively, "the Parties".

The Parties agree as follows:

1 Commencement date Agreement and position

- 1.1 Effective August 7, 2017, this employment agreement (the "Agreement") will become effective and Employee shall assume a part-time (50%), 0.5 FTE, position with the Employer in the position of Chief Scientific Officer and General Manager, Netherlands. Employee undertakes to perform all the activities as set out in Exhibit A and that can reasonably be assigned to him by or on behalf of the Employer and which are related to the Employer's business. To the best of his ability in doing so, the Employee will comply with the instructions given to him by or on behalf of the Employer.
 - 1.2 The Employee hereby resigns as a member of the Board of Directors of uniQure N.V., subject to and effective upon the appointment of his successor director at an extraordinary general meeting of shareholders of uniQure N.V. to be held on or about 14 September 2017.
 - 1.3 The Employer shall be entitled to assign other duties than the usual activities of the Employee, or to alter the position of the Employee if in the reasonable opinion of the Employer the business circumstances so require.
 - 1.4 The Employee shall not be engaged in any business activity which, in the judgment of the Employer, conflicts with Employee's ability to carry out his duties for the Company, whether or not such activity is pursued for pecuniary advantage, without the approval of the Board of Directors of uniQure N.V, other than business activities undertaken in his capacity as a general partner or venture partner of Forbion Capital Partners or its affiliated funds for up to 50% of his time. It is the mutual understanding that the Employee will resign as Managing Partner of Forbion Capital Partners by no later than June 30, 2018, it being understood that the Employee 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.
-

- 1.5 The work will be performed at the office of the Employer at Paasheuvelweg 25a (1105 BP) in Amsterdam provided, however, that the Employee shall be required to travel from time to time for business purposes. The Employer reserves the right to change the location where the work is performed after consultation with the Employee.
- 1.6 The normal working hours for a full-time, 1.0 FTE position are 40 hours per week. The working hours are normally 8.5 hours a day with a 30 minute lunch break.

2 Term and termination Agreement

- 2.1 The Agreement has been entered into for an indefinite period of time.
- 2.2 The Agreement will in any event, without notice being required, terminate as of the first day of the month following the date the Employee reaches the State pension age (AOW-gerechtigde leeftijd).
- 2.3 The Agreement can be terminated by each of the Parties with due observance of the statutory notice period of 4 months for the Employer and 2 months for the Employee.

3 Salary, bonus, equity and holiday allowance

- 3.1 The Employee's annual salary will be EUR 200,000 gross based on 0.5 FTE, including an 8% holiday allowance. The salary, excluding the holiday allowance, shall be paid in 12 equal, monthly instalments of EUR 15,432.10.
- 3.2 Once per year, in the month May, the Employer shall pay to the Employee the holiday allowance of 8% of the annual gross salary earned with the Employer in the preceding calendar year. If the Agreement commences and/or terminates during the calendar year, and/ or the Employee works on a part-time basis, the holiday allowance will be paid out pro rata.
- 3.3 The Employee shall be eligible to a bonus payment amounting to a maximum of 40% of his annual gross salary. The Employee's eligibility for a bonus payment shall be dependent on the company guidelines and is at the discretion to the Board of Directors and shall be pro-rated as appropriate to reflect a start date which is not January 1st.
- 3.4 Bonus payments, if any, will not be taken into account for the calculation of any possible severance payment upon termination of the Agreement. Employee needs to be in service on date of bonus pay out.
- 3.5 Subject to Board of Directors' approval at the next regularly scheduled uniQure N.V. Board meeting after execution of this Agreement, the Employee shall be granted an option to purchase 150,000 (one hundred fifty thousand thousand) ordinary shares of uniQure N.V., the terms of which shall reflect the standard vesting and other terms and conditions contained in the uniQure N.V.'s Amended and Restated 2014 Share Incentive Plan. Such options will be approved by the Board of Directors of uniQure N.V. not later than at its next regularly scheduled meeting and the exercise price will be the closing share price on the grant date. The Executive will be eligible for future equity grants pursuant to the Company's policies and procedures, which shall also be subject to pro-ration related to the Employee's part-time status and the employment start date.
-

4 Overtime

The Employee undertakes to work overtime at the request of the Employer. The Employer does not pay any compensation for overtime.

5 Expenses

5.1 The costs for travelling from home to office shall be compensated in accordance with the company policy.

5.2 To the extent that the Employer has given prior approval for business travels, the Employer shall reimburse reasonable travel and accommodation expenses relating to such business travel incurred by the Employee in the performance of his duties upon submission of all the relevant invoices and vouchers within 30 days following completion of the business travel.

6 Holidays

6.1 The Employee is entitled to 30 business days holiday per year or a pro rata portion thereof if the Agreement commences and/or terminates during the calendar year and/or the Employee works part-time.

6.2 The statutory holiday days (20 days of the 30 per year on full time employment) shall be forfeited after 6 months after the end of the year in which the holiday days were accrued.

6.3 The Employer shall determine the commencement and the end of the holiday in consultation with the Employee. The Employee shall take his holidays in the period that the activities best allow this.

7 Illness

In the event of illness in the sense of section 7:629 Dutch Civil Code, the Employee must report sick to the Employer as soon as possible, but no later than 9 a.m. on the first day of illness. The Employee undertakes to comply with the rules related to reporting and inspection in the case of illness, as adopted from time to time by the Employer.

8 Insurance

The Employer will comply with the obligations under the Dutch Health Care Insurance Act.

9 Pension

The Employee shall be entitled to participate in the pension scheme of the Employer following company guidelines.

10 Confidentiality obligation

10.1 Both during the term of the Agreement and after the Agreement has been terminated for any reason whatsoever, the Employee shall not make any statements in any way whatsoever to anyone whomsoever (including other personnel of the Employer, unless these should be informed of anything in connection with the work they perform for the Employer), regarding matters, activities and interests of a confidential nature related to the business of the Employer and/or the Employer's affiliates, of which the Employee became aware within the scope of his work for the Employer and the confidential nature of which he is or should be aware

("Confidential Information"). The Confidential Information includes, *inter alia*, information about the Employer's products, processes and services, including but not limited to, information relating to research, development, inventions, manufacture, purchasing, engineering, marketing, merchandising and selling.

- 10.2 For all oral and written publications by the Employee, which can or could harm the interests of the Employer, prior approval from the Employer has to be obtained. This approval shall only be refused on sincere grounds based on those interests.
- 10.3 All information exchanged via the Employer's email system is considered to be company's proprietary information and should be taken care of accordingly.
- 10.4 The Employee agrees that the confidentiality obligations set forth in this clause 10 supersede the Employee's obligations to any other company, fund or other organization with which the Employee may have a relationship ("Affiliated Entities") and that any Confidential Information that Employee receives will only be used within the scope of his employment under this Agreement or any successor agreement with Employer and will not be used during the course of his relationship, or communicated through by any means to, any Affiliated Entity.

11 Documents

The Employee is prohibited from in any way having documents and/or correspondence and/or other information carriers and/or copies thereof in his possession that belong to the Employer and/or to the Employer's affiliates, with the exception of the extent to which and as long as required for the performance of his activities for the Employer. In any event, the Employee is required, even without any request being made to that end, to return such documents and/or correspondence and/or other information carriers and/or copies thereof to the Employer immediately upon the end of the Agreement, or in the event the Employee is on non-active duty for any reason whatsoever.

12 Ban on ancillary jobs

During the term of the Agreement, without the prior written consent of the Employer, the Employee shall not accept any paid work or time-consuming unpaid work at or for third parties and will refrain from doing business for his own account, other than as provided in clause 1.4 above. For the avoidance of doubt the Employer shall not unreasonably withhold its consent for the Employee to take on any positions at third parties should the Employee fulfill such position as a Venture Partner of Forbion Capital Partners.

13 Non-competition and business relationship clause

- 13.1 Both during the term of the Agreement and for a period of one year after the Agreement has been terminated for any reason whatsoever, without the prior written consent of the Employer, the Employee shall not be engaged or involved or have any share in any manner whatsoever, directly or indirectly, whether on his own behalf or for third parties, in any enterprise which conducts activities in a field similar to or otherwise competing with that of the Employer and/or the Employer's affiliates, nor act, in any manner whatsoever, directly or indirectly, whether on his own behalf or for third parties, as an intermediary in relation to such activities. The activities contemplated by clause 1.4 shall not be deemed to be in breach of this clause 13.1.
-

- 13.2 Both during the term of the Agreement and for a period of one year after the Agreement has been terminated for any reason whatsoever, without the prior written consent of the Employer, the Employee shall not perform or have performed professional services in connection with any product or research or development or commercialization that competes with products, or research or development or commercialization of Employer, directly or indirectly, whether on his own behalf or for third parties, nor enter into contacts, in that respect, directly or indirectly, whether on his own behalf or for third parties, with clients and/or relations of the Employer and/or the Employer's affiliates and/or purchasers of products and/or services of the Employer and/or the Employer's affiliates. The activities contemplated by clause 1.4 shall not be deemed to be in breach of this clause 13.2
- 13.3 Clients and/or relations of the Employer and/or the Employer's affiliates such as set out in article 13.2 of this Agreement shall in all events mean relations of the Employer and/or the Employer's affiliates with which the Employer has or has had (business) contact in any manner whatsoever throughout the course of, or otherwise prior to the termination of, the Agreement.
- 13.4 Both during the term of the Agreement and for a period of one year after the Agreement has been terminated for any reason whatsoever, without the prior written consent of the Employer, the Employee shall refrain from becoming engaged or involved in any manner whatsoever, directly or indirectly, whether on his own behalf or for third parties, in actively enticing away, taking (or causing to have taken) into employment, nor make use of, in any manner whatsoever, directly or indirectly, whether on his own behalf or for third parties, the type of work of employees or persons who in a period of one year prior to the termination of the Agreement of the Employee are or have been in the employment of the Employer and/or the Employer's affiliates.
- 13.5 Employee acknowledges and agrees to adhere to this clause as the Employer has a serious business interest in binding the Employee to the non-competition and business relationship clause, due to the fact that (i) within the organization of the Employer competition-sensitive information as well as confidential information related to the Employer and its clients and relations, such as but not limited to products, or research or development or commercialization of Employer ("Sensitive Business Information") are available and (ii) in the position of **Chief Scientific Officer and GM, Amsterdam** the Employee has access to this Sensitive Business Information and/or will become aware of this Sensitive Business Information and/or will maintain (commercial) contacts with clients, suppliers, competitors etc. Given the aforesaid considerations (i) and (ii) in this clause, combined with the education and capacities of the Employee, the Employer has a well-founded fear that its business interest will be harmed substantially if the Employee performs competing activities as set forth in clauses 13.1 up to and including 13.5 of the Agreement within a period of 12 months after termination of the Agreement.
- 14 Intellectual and industrial property**
- 14.1 The Employer is or will be considered to be, to the fullest extent allowed by law, the maker/producer/designer/breeder of all that which is made, created, improved, produced, designed, invented or discovered by the Employee during his activities performed for the Employer (the **Works**).
-

- 14.2 The Employee is obliged to fully and comprehensibly disclose all Works to the Employer in writing immediately after they are created or after the creation becomes known to the Employee, and in any case at the request of the Employer.
- 14.3 The Employee hereby transfers and assigns all his rights to and in connection with the Works to the Employer in advance.
- 14.4 The Employee is obliged, at first request of the Employer, to transfer and assign to the Employer all rights to and in connection with the Works that do not belong to the Employer by operation of law (*van rechtswege*), and that are not transferred to the Employer pursuant to article 14.3 of this Agreement. This concerns all rights, anywhere in the world, to and arising from or in connection with the Works. This obligation of the Employee remains in force even after the end of this Agreement.
- 14.5 The Employee agrees to perform, to the extent necessary and/or at the request of the Employer, such further acts as may be necessary or desirable to apply for, obtain and/or maintain protection for the Works, *inter alia* by means of the establishment of intellectual and industrial property rights. The Employee hereby grants permission and power of attorney to the Employer to the extent necessary to carry out every required act on behalf of the Employee to obtain protection for the Works, or to transfer the Works and any rights relating thereto, to the Employer. The Employer will compensate the reasonable costs made in respect hereof, in so far as the payment that the Employee receives pursuant to article 3.1 of this Agreement cannot be considered as compensation for such costs. This obligation of the Employee remains in force even after the end of the Agreement.
- 14.6 The Employee acknowledges that the payment ex article 3.1 of this Agreement includes a reasonable compensation for any possible deprivation of any intellectual and industrial property rights. To the extent legally possible, the Employee hereby waives his right to any additional compensation with respect to the Works.

15 Gifts

In connection with the performance of his duties, the Employee is prohibited from accepting or stipulating, either directly or indirectly, any commission, reimbursement or payment, in whatever form, or gifts from third parties other than those covered under article 1.4. The foregoing does not apply to standard promotional gifts having little monetary value.

16 Penalty clause

In the event the Employee acts in violation of any of the obligations under the articles 10 through 15 of this Agreement, the Employee shall, contrary to section 7:650 paragraphs 3, 4 and 5 Dutch Civil Code, without notice of default being required, forfeit to the Employer for each such violation, a penalty in the amount of EUR 10.000,00 as well as a penalty of EUR 1.000,00 for each day such violation has taken place and continues. Alternatively, the Employer will be entitled to claim full damages.

17 Transfer of an undertaking

The Employee shall remain under the obligation to adhere the set out in the articles 10 through 16 of this Agreement vis-à-vis the Employer, if the enterprise of the Employer or a part thereof is transferred to a third party within the meaning of section 7:662 and onwards Dutch Civil

Code and this Agreement terminates before or at the time of such transfer, whereas in the event of continuation of the Agreement the Employee would have entered the employment of the acquirer by operation of law.

18 Other arrangements

Subject to the provisions in this Agreement, the arrangements related to employment conditions adopted by the Employer from time to time, as laid down in the Employee Handbook are applicable. A copy of these arrangements has been provided to the Employee. By signing this agreement, the Employee acknowledges to have received and understood the Employee Handbook and the Insider Trading Policy.

19 Employment costs regulation

The conditions of employment costs regulation determined by the Employer apply. In this context, the Employer reserves the right at its sole discretion to modify certain fringe benefits, without any compensation in return.

20 Amendment clause

20.1 The Employer reserves the right to unilaterally amend the Agreement and the arrangements referred to in article 18 of this Agreement if it has such a serious interest in that respect entailing that the interests of the Employee must yield to that in accordance with standards of reasonableness and fairness.

20.2 The Employer reserves the right to unilaterally amend the Agreement and the arrangements referred to in article 18 of this Agreement in the event of a relevant amendment of the law.

21 Applicable law, no collective labour agreement

21.1 This Agreement is governed by Dutch law.

21.2 The Agreement is not subject to any collective labour agreement.

uniQure biopharma B.V.

uniQure biopharma B.V.

/s/ Matthew Kapusta
By: Matthew Kapusta
Title: CEO

By:
Title:

Employee

/s/ Sander van Deventer
By: Sander van Deventer

Exhibit A

Reporting to Chief Executive Officer, the Chief Scientific Officer (CSO) and General Manager, Netherlands will be responsible for the company's research, scientific and technology platform strategy and activities in support of the company's portfolio strategy, including basic and applied research projects, as well as the development of new processes, technologies or products.

Additionally, the CSO will also provide guidance on scientific-related matters within the company and represent uniQure at scientific/medical conferences, as well as with investors and regulatory agencies.

Job Responsibilities:

- Develop, refine and execute uniQure's research, scientific and technology platform strategy that supports and enhances the corporate long-term plan;
 - Effectively communicate a vision and plan related to new product development, as well as scientific and technological matters;
 - In collaboration with the CEO, Commercial leader and Business Development leader, define a process and criteria for identifying new targets, indications and potential gene therapy product candidates;
 - Provide scientific guidance on strategic and operating decisions, setting strategy, and performance goals;
 - Regularly reporting to the Board and other members of the organization to ensure transparency regarding the progress of research and platform development programs;
 - Work closely with the Chief Operating Officer, Chief Medical Officer and other key leaders to ensure execution on a corporate, global strategy related to R&D and technology/platform development;
 - Provide strong scientific leadership for the uniQure research, nonclinical and emerging technology development teams;
 - Lead the effort to translate discovery research into clinical-ready product candidates;
 - Establish and/or help to maintain relationships with KOLs and academic institutions, and serve as a key liaison between the company and its external scientific advisors and the investment community;
 - Identify collaboration opportunities to gain access to key capabilities and provide leadership in managing such collaborations, including uniQure's key collaboration with BMS;
 - Provide scientific and clinical expertise in support of product and clinical development activities;
 - Participate in business development and in-licensing activities, including due diligence activities as required;
 - Participate in leadership team meetings, Board meetings and other key operating mechanisms required of senior management and by the Chief Executive Officer;
 - Make and attend scientific presentations, and participate in key scientific and medical conferences;
 - Develop budgets for relevant functional responsibilities, subject to approval by the Chief Executive Officer and Principal Financial Officer, and ensure execution within approved targets;
 - Foster and develop an innovative and productive organization of talented scientists, including the management, motivation, recruitment and evaluation of personnel; and
 - Plan, organize, lead and control the daily activities of uniQure's operation in Amsterdam
-

EMPLOYMENT AGREEMENT

THIS EMPLOYMENT AGREEMENT (this “Agreement”) is made and entered into as of July 10, 2017 (the “Effective Date”), by and between uniQure, Inc., 113 Hartwell Avenue, Lexington, MA 02421 (the “Company”) and Scott T. McMillan (the “Executive”), 109 Hayward Road, Acton, MA 01720.

WITNESSETH:

WHEREAS, the Company desires to employ Executive, and Executive desires to be employed by the Company, each upon the terms set forth herein.

NOW, THEREFORE, in consideration of the mutual promises and covenants contained herein and intending to be legally bound hereby, the Company and Executive agree as follows.

1. Employment. The Company hereby agrees to employ Executive, and Executive hereby accepts employment by the Company, as a full-time employee for the period and upon the terms and conditions contained in this Agreement.
2. Term. Executive’s term of employment with the Company under this Agreement shall begin on August 7, 2017 (the “Start Date”) and shall continue in force and effect from year to year unless terminated earlier in accordance with Section 19 (the “Term”).
3. Position and Duties. During the Term, Executive shall serve the Company as its Chief Operations Officer, reporting directly to the uniQure Chief Executive Officer (the “CEO”).

The initial primary focus of the Chief Operations Officer will be:

- § Responsible for designing uniQure’s operations strategy and tactical execution in the context of uniQure’s overall strategy
- § Responsible for timely and compliant execution of the related activities in the relevant areas and identification of opportunities for continuous improvement. This includes compliance with both GMP regulations, as well as e.g. environmental and safety regulations
- § Responsible for managing the technology transfer process between the Amsterdam and Lexington facilities
- § Responsible for defining, maintaining and adherence to the Operations budget for expenses, capital investment and human resources
- § Responsible for all investment and maintenance activities for uniQure’s premises, utilities and equipment, either executed by internal resources and/or by third parties
- § Responsible for managing (in)direct reports, including appraisals, development/succession planning and personnel actions

Scott T. McMillan

Employment Agreement

Page 1

Initials: _____

- § Responsible for definition, implementation, maintenance and continued improvement of processes and systems, supported by meaningful Key Performance Indicators (KPI's)
- § Interacts with staff of other disciplines, such as Finance, Research, Human Resources and Clinical Development to ensure efficient day-to-day cooperation and success for the business
- § Interaction with Commercial and Collaboration Partners for management of forecasting and production planning/scheduling activities

4. Commencing on the Start Date and during the Term, Executive shall devote full business time, best efforts, skill, knowledge, attention and energies to the advancement of the Company's business and interests and to the performance of Executive's duties and responsibilities as an employee of the Company. Executive shall abide by the rules, regulations, instructions, personnel practices and policies of the Company and any changes therein that may be adopted from time to time by the Company.

5. Notwithstanding the foregoing, Executive may engage in civic and charitable organizations and manage his or her personal and business affairs during normal business hours provided that such activities do not, individually or collectively, interfere with the performance of his or her duties hereunder. Commencing on the Start Date and during the Term, and subject to the provisions of Paragraph 4 above, Executive shall not be engaged in any business activity which, in the judgment of the Company, conflicts with Executive's duties hereunder, whether or not such activity is pursued for pecuniary advantage. Should Executive wish to provide any services to any other person or entity other than the Company or to serve on the board of directors of any other entity or organization, Executive shall submit a written request to the Company for consideration and approval by the Company, which approval shall not unreasonably be withheld. If the Company later makes a reasonable, good faith determination that Executive's continued service on another entity's board would be detrimental to the Company, it will give Executive thirty (30) days' written notice that it is revoking the original approval, and Executive will resign from the applicable board within thirty (30) days after receipt of such notice.

6. Location. Executive shall perform the services hereunder from the Company's USA headquarters at 113 Hartwell Avenue, Lexington MA, USA; provided, however, that Executive shall be required to travel from time to time for business purposes.

7. Compensation and Benefits.

- (a) *Base Salary.* For all services rendered by Executive under this Agreement, the Company will pay him or her a base salary at the annual rate of \$360,000 (three hundred and sixty thousand dollars), which shall be reviewed annually by the CEO for adjustment (the base salary in effect at any time, the "Base Salary"). In order to be eligible for an increase in Base Salary applicable to the year following the year in which the Start Date falls, Executive's Start Date must be before October 1st in any given year. Any merit increase would be pro-rated for the 2017 calendar year. Executive's Base Salary shall be paid in bi-weekly installments, less withholdings as required by law and deductions authorized by Executive, and payable pursuant to the Company's regular payroll practices in effect at the time.

Scott T. McMillan

Employment Agreement

Page 2

Initials: _____

(b) Discretionary Bonus. Following the end of each calendar year and subject to the approval of the Company, Executive shall be eligible for a retention and performance bonus of forty percent (40%) of the annual Base Salary based on performance and the Company's performance and financial condition during the applicable calendar year, as determined by the Company in its sole discretion. In any event, Executive must be an active employee of the Company on the date the bonus is distributed in order to be eligible for and to earn any bonus, as it also serves as an incentive to remain employed by the Company. In order to be eligible for a bonus which is paid in the year following the year in which the Start Date falls, Executive's Start Date must be before October 1st in any given year. Any bonus will not be pro-rated for the 2017 calendar year.

8. Equity. Subject to Board of Directors approval at the next regularly scheduled uniQure N.V. Board meeting after execution of this Agreement, Executive shall be granted an option to purchase 150,000 (one hundred and twenty-five thousand) ordinary shares of the Company, the terms of which shall reflect the standard vesting and other terms and conditions contained in the uniQure N.V.'s Amended and Restated 2014 Share Incentive Plan. Such options will be approved by the Board of Directors of uniQure N.V. not later than at its next regularly scheduled meeting and the exercise price will be the closing share price on the grant date. If the Board fails to make the grant at such regularly scheduled meeting, it shall be deemed a Good Reason event under Section 19(f) hereof. The Executive will be eligible for future equity grants pursuant to the Company's policies and procedures.

9. Benefits. Executive is eligible to participate in any and all benefit programs that the Company establishes and makes available to its employees from time to time, provided that Executive is eligible under (and subject to all provisions of) the plan documents that govern those programs. These include medical, dental and disability insurances. Benefits are subject to change at any time in the Company's sole discretion.

10. Paid Time Off and Holidays. Executive is eligible for four (4) weeks of paid vacation per calendar year (pro-rated for 2017) to be taken at such times as may be approved in advance by the Company. Executive is also entitled to all paid holidays observed by the Company in the United States. Executive shall have all rights, including the right to potentially earn more than four (4) weeks of paid vacation per year, and be subject to all obligations and responsibilities with respect to paid time off and holidays as are set forth in the Company's employee manual or other applicable policies and procedures.

11. 401(k) Plan. Subject to Section 9 (benefits).

12. Expense Reimbursement. During the Term, Executive shall be reimbursed by the Company for all necessary and reasonable expenses incurred by Executive in connection with the performance of Executive's duties hereunder (including business trips to the uniQure Amsterdam headquarters). Executive shall keep an itemized account of such expenses, together with vouchers and/or receipts verifying the same, and submit for reimbursement on a monthly basis. Any such expense reimbursement will be made in accordance with the Company's travel and expense policies governing reimbursement of expenses as are in effect from time to time.

Scott T. McMillan

Employment Agreement

Page 3

Initials: _____

13. Withholding. All amounts set forth in this Agreement are on a gross, pre-tax basis and shall be subject to all applicable federal, state, local and foreign withholding, payroll and other taxes, and the Company may withhold from any amounts payable to Executive (including any amounts payable pursuant to this Agreement) in order to comply with such withholding obligations.

14. IP and Restrictive Covenants. Executive's employment is contingent upon Executive's execution of the Company's Confidentiality, Developments, and Restrictive Covenants Agreement, attached as Exhibit A to this Agreement.

15. At-Will Employment. This Agreement shall not be construed as an agreement, either express or implied, to employ Executive for any stated term, and shall in no way alter the Company's policy of employment at-will, under which both the Company and Executive remain free to end the employment relationship for any reason, at any time, with or without cause or notice. Similarly, nothing in this Agreement shall be construed as an agreement, either express or implied, to pay Executive any compensation or grant Executive any benefit beyond the end of employment with the Company, other than as set out elsewhere in this Agreement.

16. Conflicting Agreements. Executive acknowledges and represents that by executing this Agreement and performing Executive's obligations under it, Executive has disclosed and provided copies to the Company of any and all confidentiality or restrictive covenant agreements that Executive is a party or is bound, to that could limit or prohibit the full performance of Executive's duties to the Company.

17. No Prior Representations. This Agreement and its exhibits constitute all the terms of Executive's hire and supersede all prior representations or understandings, whether written or oral, relating to the terms and conditions of Executive's employment.

18. Change of Control. In the event of a Change of Control as defined below, the vesting conditions that may apply to any equity held by Executive pursuant to this Agreement and the Company's Amended and Restated 2014 Share Incentive Plan will be automatically waived and all the equity will be deemed to be fully exercisable commencing on the date of the Change of Control and ending on the eighteen (18) month anniversary of the Change of Control. For purposes of this Agreement, "Change of Control" shall mean the date on which any of the following events occurs:

- (a) any "person," as such term is used in Sections 13(d) and 14(d) of the Securities Exchange Act of 1934, as amended (the "Act") (other than the Company, any of its subsidiaries, or any trustee, fiduciary or other person or entity holding securities under any employee benefit plan or trust of the Company or any of its subsidiaries), together with all "affiliates" and "associates" (as such terms are defined in Rule 12b-2 under the Act) of such person, shall become the "beneficial owner" (as such term is defined in Rule 13d-3 under the Act), directly or indirectly, of securities of the Company representing forty (40) percent or more of the combined voting power of the Company's then outstanding securities having the right to vote in an election of the Board ("Voting Securities") (in such case other than as a result of an acquisition of securities directly from the Company); or

Scott T. McMillan

Employment Agreement

Page 4

Initials: _____

- (b) a majority of the members of the Board is replaced during any 12-month period by directors whose appointment or election is not endorsed by a majority of the members of the Board before the date of the appointment or election; or
- (c) the consummation of (i) any consolidation or merger of the Company where the stockholders of the Company, immediately prior to the consolidation or merger, would not, immediately after the consolidation or merger, beneficially own (as such term is defined in Rule 13d-3 under the Act), directly or indirectly, shares representing in the aggregate more than fifty (50) percent of the voting shares of the Company issuing cash or securities in the consolidation or merger (or of its ultimate parent corporation, if any), or (ii) any sale or other transfer (in one transaction or a series of transactions contemplated or arranged by any party as a single plan) of all or substantially all of the assets of the Company.

19. Termination. The Term shall continue until the termination of Executive's employment with the Company as provided below.

- (a) *Events of Termination.* Executive's employment, Base Salary and any and all other rights of Executive under this Agreement or otherwise as an employee of the Company will terminate:
 - (i) upon the death of Executive;
 - (ii) upon the Disability of Executive (immediately upon notice from either party to the other). For purposes hereof, the term "Disability" shall mean an incapacity by accident, illness or other circumstances which renders Executive mentally or physically incapable of performing the duties and services required of Executive hereunder on a full-time basis for a period of at least 120 consecutive days.
 - (iii) upon termination of Executive for Cause;
 - (iv) upon the resignation of employment by Executive without Good Reason (upon thirty (30) days' prior written notice);
 - (v) upon termination by the Company for any reason other than those set forth in Sections 19(a)(i) through 19(a)(iv) above;
 - (vi) upon voluntary resignation of employment by Executive for Good Reason as described in Section 19(f), below;
 - (vii) upon a Change of Control Termination as described in Section 19(g), below.
- (b) In the event Executive's termination occurs pursuant to Sections 19(a)(i) - (iv) above, Executive will be entitled only to the Accrued Benefits through the termination date. The Company will have no further obligation to pay any compensation of any kind (including,

Scott T. McMillan

Employment Agreement

Page 5

Initials: _____

without limitation, any bonus or portion of a bonus that otherwise may have become due and payable to Executive with respect to the year in which such termination date occurs), or severance payment of any kind, unless otherwise provided herein. For purposes of this agreement, Accrued Benefits shall mean (i) payment of Base Salary through the termination date, (ii) any payments or benefits under the Company's benefit plans that are vested, earned or accrued prior to the termination date (including, without limitation, earned but unused vacation); and (iii) payment of unreimbursed business expenses incurred by Executive.

- (c) For purposes of this Agreement, "Cause" shall mean the good faith determination by the Company (which determination shall be conclusive), after written notice from the Company to Executive that one or more of the following events has occurred and stating with reasonable specificity the actions that constitute Cause and the specific reasonable cure (related to subsections (i) and (viii) below):
- (i) Executive has willfully or repeatedly failed to perform Executive's material duties and such failure has not been cured after a period of thirty (30) days' written notice;
 - (ii) any reckless or grossly negligent act by Executive having the foreseeable effect of injuring the interest, business or reputation of the Company, or any of its parents, subsidiaries or affiliates in any material respect;
 - (iii) Executive's evidenced use of any illegal drug, or illegal narcotic, or excessive amounts of alcohol (as determined by the Company in its reasonable discretion) on Company property or at a function where Executive is working on behalf of the Company;
 - (iv) the indictment on charges or conviction for (or the procedural equivalent of conviction for), or entering of a guilty plea or plea of no contest with respect to a felony;
 - (v) the conviction for (or the procedural equivalent of conviction for), or entering of a guilty plea or plea of no contest with respect to a misdemeanor which, in the Company's reasonable judgment, involves moral turpitude, deceit, dishonesty or fraud, except that, in the event that Executive is indicted on charges for a misdemeanor, the Company may elect, in its sole discretion, to place Executive on administrative garden leave with or without continuation of full compensation and benefits under this Agreement during the pendency of the proceedings;
 - (vi) conduct by or at the direction of Executive constituting misappropriation or embezzlement of the property of the Company, or any of its parents or affiliates (other than the

occasional, customary and *de minimis* use of Company property for personal purposes);

- (vii) a material breach by Executive of a fiduciary duty owing to the Company, including the misappropriation of (or attempted misappropriation of) a corporate opportunity or undisclosed self-dealing;
 - (viii) a material breach by Executive of any material provision of this Agreement, any of the Company's written employment policies or Executive's fiduciary duties to the Company, which breach, if curable, remains uncured for a period of thirty (30) days after receipt by Executive of written notice of such breach from the Company, which notice shall contain a reasonably specific description of such breach and the specific reasonable cure requested by the Board; and
 - (ix) any material breach of Executive's Confidentiality, Developments, and Restrictive Covenants Agreement.
- (d) The definition of Cause set forth in this Agreement shall govern for purposes of Executive's equity compensation and any other compensation containing such a concept.
- (e) *Notice Period for Termination Under Section 19(a)(iv).* Upon a termination of Executive under Section 19(a)(iv), during the notice period the Company may, in its sole discretion, relieve Executive of all of Executive's duties, responsibilities, and authority, may restrict Executive's access to Company property, and may take other appropriate measures deemed necessary under the circumstances.
- (f) *Termination by Executive for Good Reason.* During the Term, Executive may terminate this Agreement at any time upon thirty (30) days' written notice to the Company for "Good Reason." For purposes of this Agreement, "Good Reason" shall mean that Executive has complied with the "Good Reason Process" (hereinafter defined) following the occurrence of any of the following actions undertaken by the Company without Executive's express prior written consent: (i) the material diminution in Executive's responsibilities, authority and function; (ii) a material reduction in Executive's Base Salary, provided, however, that Good Reason shall not be deemed to have occurred in the event of a reduction in Executive's Base Salary which is pursuant to a salary reduction program affecting the CEO and all or substantially all other senior management employees of the Company and that does not adversely affect Executive to a greater extent than other similarly situated employees; provided, however that such reduction may not exceed twenty (20%) percent; (iii) a material change in the geographic location at which Executive provides services to the Company (i.e., outside a radius of fifty (50) miles from Lexington, Massachusetts) (each a "Good Reason Condition").

"Good Reason Process" shall mean that: (i) Executive has reasonably determined in good faith that a Good Reason Condition has occurred; (ii) Executive has notified the Company in writing of the first occurrence of the Good Reason Condition within 60 days of the first occurrence of such condition; (iii) Executive has cooperated in good faith with the Company's efforts, for a period not less than thirty (30) days following such notice (the "Cure Period"), to remedy the condition; (iv) notwithstanding such efforts, the Good Reason Condition continues to exist; and (v) Executive terminates employment within sixty (60) days after the end of the Cure Period. If the Company cures to Executive's satisfaction (not unreasonably withheld) the Good Reason condition during the Cure Period, Good Reason shall be deemed not to have occurred.

- (g) *Termination As A Result of a Change Of Control.* For purposes of this Agreement, "Change of Control Termination" shall mean any of the following:
- (i) Any termination by the Company of Executive's employment, other than for Cause (as defined in Section 19(c), above), that occurs within twelve (12) months after the Change of Control; or
 - (ii) Any resignation by Executive for Good Reason (as defined in Section 19(f), above), that occurs within twelve (12) months after the Change of Control.
 - (iii) For purposes of this Section 19(g), "Change of Control" shall have the same meaning as defined above in Section 18.
- (h) *Separation Benefits.* Should Executive experience a termination of employment during the Term pursuant to Section 19(a)(v), (vi), or (vii) above, in addition to the Accrued Benefits Executive shall also be entitled to a lump sum severance payment equal to 100% of the annual Base Salary. To avoid duplication of severance payments, any amount paid under this subsection shall be offset against any severance amounts that may be owed by the Company to Executive pursuant to the Company's Change of Control guidelines.

20. General Release of Claims. Notwithstanding any provision of this agreement, all severance payments and benefits described in Section 19 of this Agreement (except for payment of the Accrued Benefits) are conditioned upon the execution, delivery to the Company, and expiration of any applicable revocation period without a notice of revocation having been given by Executive, all by the 30th day following the termination date of a General Release of Claims by and between Executive (or Executive's estate) and the Company in the form attached as Exhibit B to this Agreement. (In the event of Executive's death or incapacity due to Disability, the release will be revised for signature accordingly.) Provided any applicable timing requirements set forth above have been met, the payments and benefits will be paid or provided to Executive as soon as administratively practicable (but not later than forty-five (45) days) following the date Executive signs and delivers the General Release to the Company and any applicable revocation period has expired without a

Scott T. McMillan

Employment Agreement

Page 8

Initials: _____

notice of revocation having been given. Any severance or termination pay will be the sole and exclusive remedy, compensation or benefit due to Executive or Executive's estate upon any termination of Executive's employment (without limiting Executive's rights under any disability, life insurance, or deferred compensation arrangement in which Executive participates or at the time of such termination of employment or any Option Agreements or any other equity agreements to which Executive is a party). If such 45-day period spans two calendar years, payment will be paid after such 45-day period and revocation period have expired.

21. Certain Company Remedies. Executive acknowledges that Executive's promised services and covenants are of a special and unique character, which give them peculiar value, the loss of which cannot be reasonably or adequately compensated for in an action at law, and that, in the event there is a breach hereof by Executive, the Company will suffer irreparable harm, the amount of which will be impossible to ascertain. Accordingly, the Company shall be entitled, if it so elects, to institute and prosecute proceedings in any court of competent jurisdiction, either at law or in equity, to obtain damages for any breach of this Agreement, or to enjoin Executive from committing any act in breach of this Agreement. The remedies granted to the Company in this Agreement are cumulative and are in addition to remedies otherwise available to the Company at law or in equity.

22. Indemnification.

- (a) The Company agrees that Executive shall be entitled to indemnification to the fullest extent permitted by Delaware law and under the Company's articles of incorporation, bylaws and any other corporate-related plan, program or policy. In addition, as soon as reasonably practicable following the Start Date and for a period of at least three (3) years after Executive's termination of employment, the Company shall maintain a directors and officers liability insurance policy under which Executive shall be included as a "Covered Person."
- (b) In addition, and for the sake of clarity, the Company hereby specifically agrees that (i) if Executive is made a party, or is threatened to be made a party, to any "Proceeding" (defined as any threatened or actual action, suit or proceeding whether civil, criminal, administrative, investigative, appellate or other) by reason of the fact that (1) Executive is or was an employee, officer, director, agent, consultant or representative of the Company, or (2) is or was serving at the request of the Company or any of its affiliates as employee, officer, director, agent, consultant or representative of another person, or (ii) if any "Claim" (defined as any claim, demand, request, investigation, dispute, controversy, threat, discovery request or request for testimony or information) is made, or threatened to be made, that arises out of or relates to Executive's service in any of the foregoing capacity or to the Company, then Executive shall be indemnified and held harmless by the Company to the fullest extent permitted by applicable law, against any and all costs, expenses, liabilities and losses (including, without limitation, attorney's fees, judgments, interest, expenses of investigation, penalties, fines, taxes or penalties and amounts paid or to be paid in settlement) incurred or suffered by Executive in connection therewith, except with respect to any costs, expenses, liabilities or

Scott T. McMillan

Employment Agreement

Page 9

Initials: _____

losses (A) that were incurred or suffered as a result of Executive's willful misconduct, gross negligence or knowing violation of any written agreement between Executive and the Company, or (B) that a court of competent jurisdiction determines to have resulted from Executive's knowing and fraudulent acts; provided, however, that the Company shall provide such indemnification only if (I) notice of any such Proceeding is given promptly to the Company, by Executive; (II) the Company is permitted to participate in and assume the defense of any such Proceeding; (III) such cost, expense, liability or loss results from the final judgment of a court of competent jurisdiction or as a result of a settlement entered into with the prior written consent of the Company; and (IV) in the case of any such Proceeding (or part thereof) initiated by Executive, such Proceeding (or part thereof) was authorized in advance in writing by the Company. Such indemnification shall continue even if Executive has ceased to be an employee, officer, director, agent, consultant or representative of the Company or an affiliate thereof until all applicable statute of limitations have expired, and shall inure to the benefit of Executive's heirs, executors and administrators. The Company shall pay directly or advance to Executive all costs and expenses incurred by Executive in connection with any such Proceeding or Claim (except for Proceedings brought by the Company against Executive for claims other than shareholder derivative actions) within 30 days after receiving written notice requesting such an advance. Such notice shall include, to the extent required by applicable law, an undertaking by Executive to repay the amount advanced if Executive was ultimately determined not to be entitled to indemnification against such costs and expenses

23. Miscellaneous.

- (a) *Right to Offset.* The Company may offset any undisputed amounts Executive owes the Company or its affiliates at the time of Executive's termination of employment (including any payment of Accrued Benefits or separation pay), except for secured or unsecured loans, against any amounts the Company owes Executive hereunder including, but not limited to, any wages, accrued vacation and bonuses, which Executive acknowledges and agrees would constitute a valid offset pursuant to any state or federal law (including the Massachusetts Payment of Wages Statute, M.G.L. c. 149 § 148 et. seq.).

- (b) *Cooperation.* Executive agrees that, during and after Executive's employment with the Company, subject to reimbursement of Executive's reasonable expenses, Executive will cooperate fully with the Company and its counsel with respect to any matter (including, without limitation, litigation, investigations, or governmental proceedings) in which Executive was in any way involved during Executive's employment with the Company. Executive shall render such cooperation in a timely manner on reasonable notice from the Company, and at such times and places as reasonably acceptable to Executive and the Company. The Company, following Executive's termination of employment, exercises commercially reasonable efforts

Scott T. McMillan

Employment Agreement

Page 10

Initials: _____

to schedule and limit its need for Executive's cooperation under this paragraph so as not to interfere with Executive's other personal and professional commitments.

- (c) *Company Documents and Property.* Upon termination of Executive's employment with the Company, or at any other time upon the request of Company, Executive shall forthwith deliver to Company any and all documents, notes, notebooks, letters, manuals, prints, drawings, block diagrams, photocopies of documents, devices, equipment, keys, security passes, credit cards, hardware, data, databases, source code, object code, and data or computer programming code stored on an optical or electronic medium, and any copies thereof, in the possession of or under the control of Executive that embodies any confidential information of the Company. Executive agrees to refrain from purging or deleting data from any Company-owned equipment, including email systems, in connection with Executive's termination. To the extent that Executive possesses any data belonging to Company on any storage media owned by Executive (for example, a home computer's hard disk drive, portable data storage device, etc.), Executive agrees that Executive will work cooperatively with the Company to return such data and ensure it is removed from Executive's devices in a manner that does not adversely impact any personal data. Executive agrees not to take any steps to delete any Company data from any device without first obtaining Company's written approval. Executive agrees to cooperate with Company if Company requests written or other positive confirmation of the return or destruction of such data from any personal storage media. Nothing herein shall be deemed to prohibit Executive from retaining (and making copies of): (i) Executive's personal non-business-related correspondence files; or (ii) documents relating to Executive's personal compensation, benefits, and obligations and documents reasonably necessary to prepare personal income tax returns.
- (d) *Waivers.* No waiver of any provision will be effective unless made in writing and signed by the waiving party. The failure of any party to require the performance of any term or obligation of this Agreement does not prevent subsequent enforcement of that term or obligation. The waiver by any party of any breach of this Agreement does not waive any subsequent breach.
- (e) *Governing Law; Consent to Exclusive Jurisdiction and Venue/Jury Waiver.* This Agreement and all questions relating to its validity, interpretation, performance and enforcement (including, without limitation, provisions concerning limitations of actions), shall be governed by and construed in accordance with the laws of the Commonwealth of Massachusetts (notwithstanding any conflict-of-laws doctrines of such state or other jurisdiction to the contrary), and without the aid of any canon, custom or rule of law requiring construction against the draftsman. The parties hereby consent and submit to the exclusive jurisdiction of the federal and state courts in the Commonwealth of Massachusetts, and to exclusive venue in any

Massachusetts federal court and/or Massachusetts state court located in Suffolk County, for any dispute arising from this Agreement. The parties further acknowledge and agree that any such dispute shall be tried by a Judge alone, and both parties hereby waive and forever renounce the right to a trial before a civil jury. Executive hereby agrees that he will neither commence or prosecute, nor assist in any way another person or entity to commence or prosecute, any legal action or other proceeding (including but not limited to a declaratory judgment action) against the Company concerning a dispute arising from or relating to this Section in any forum or jurisdiction other than the state and federal courts in the Commonwealth of Massachusetts. Executive further agrees that, in the event Executive disregards this clause, the Company shall be entitled to recover its reasonable attorneys' fees and other costs incurred in staying, transferring, dismissing or otherwise defending such out-of-state action or proceeding, regardless of whether such fees and costs are incurred in the forum where Executive (or another person or entity, as applicable), commenced the action or in a Massachusetts forum, and without regard to whether the Company prevails in its efforts to enforce this covenant.

- (f) *Notices.* Any notices, requests, demands, and other communications described in this Agreement are sufficient if in writing and delivered in person or sent postage prepaid, by certified or registered U.S. mail or by FedEx/UPS to Executive at Executive's last known home address and a copy by e-mail to Executive, or in the case of the Company, to the attention of the General Counsel, copy to the CEO, at the main office of uniQure, Inc. Any notice sent by U.S. mail shall be deemed given for all purposes 72 hours from its deposit in the U.S. mail, or the next day if sent by overnight delivery.
- (g) *Successors and Assigns.* Executive may not assign this Agreement, by operation of law or otherwise, without the Company's prior written consent. Without the Company's consent, any attempted transfer or assignment will be void and of no effect. The Company may assign its rights under this Agreement if the Company consolidates with or merges into any other entity, or transfers substantially all of its properties or assets to any other entity, provided that such entity expressly agrees to be bound by the provisions hereof. This Agreement will inure to the benefit of and be binding upon the Company and Executive, their respective successors, executors, administrators, heirs, and permitted assigns.
- (h) *Counterparts; Facsimile.* This Agreement may be executed in two or more counterparts, each of which shall be an original and all of which together shall constitute one and the same instrument. This Agreement may be executed by facsimile transmission, PDF, electronic signature or other similar electronic means with the same force and effect as if such signature page were an original thereof.

Scott T. McMillan

Employment Agreement

Page 12

Initials: _____

- (i) *Severability.* The provisions of this Agreement are independent of and separable from each other, and no provision shall be affected or rendered invalid or unenforceable by virtue of the fact that for any reason any other provision or provisions may be invalid or unenforceable in whole or in part.
- (j) *Enforceability.* If any portion or provision of the Agreement is declared illegal or unenforceable by a court of competent jurisdiction, the remainder of the Agreement will not be affected, and each remaining portion and provision of this Agreement will be valid and enforceable to the fullest extent permitted by law.
- (k) *Survival.* Sections 14, 20, 21, and the Company's Confidentiality, Developments, and Restrictive Covenants Agreement (Exhibit A) and all other provisions necessary to give effect thereto, shall survive the termination of Executive's employment for any reason.
- (l) *Entire Agreement; Amendment.* This Agreement contains the entire understanding among the parties hereto with respect to the subject matter hereof, and supersedes all prior and contemporaneous agreements and understandings, inducements or conditions, express or implied, oral or written, between the parties hereto (including without limitation any prior employment agreements between the parties hereto); provided, however, that any agreements referenced in this Agreement or executed herewith are not superseded. The express terms hereof control and supersede any course of performance and/or usage of the trade inconsistent with any of the terms hereof. This Agreement may be amended or modified only by a written instrument signed by Executive and by a duly authorized representative of the Company.
- (m) *Section Headings.* The section headings in this Agreement are for convenience only, form no part of this Agreement and shall not affect its interpretation.

[This space intentionally left blank.]

Scott T. McMillan

Employment Agreement

Page 13

Initials: _____

IN WITNESS WHEREOF, the parties have executed and delivered this Agreement as of the date first above written.

uniQure, Inc.

By: /s/ Matt Kapusta

Name: Matt Kapusta
Title: Chief Executive Officer

EXECUTIVE

/s/ Scott T. McMillan
Scott T. McMillan

**EMPLOYMENT AGREEMENT PURSUANT TO
SECTION 7:610 (et seq.) of the Dutch Civil Code
(DCC)**

[July 15, 2017]

- (1) **uniQure biopharma B.V.**, a private company with limited liability (*besloten vennootschap met beperkte aansprakelijkheid*), with registered office at Amsterdam and principal place of business at Paasheuvelweg 25a, (1105 BP) Amsterdam (the **Employer**); and
- (2) **Christian Klemt** born on October 8, 1972, residing at the address Heemsteedse Dreef 275 at (2102 KJ) Heemstede (the **Employee**).

The Employer and the Employee are hereinafter referred to jointly as **Parties** and individually as **Party**.

Whereas the Parties entered into an employment agreement effective September 1, 2015 by which the Employee assumed the responsibilities of Global Controller ("Initial Agreement");

Whereas terms and condition of the Initial Agreement do no longer reflect the actual situation;

Whereas the Parties desire to replace the Initial Agreement by this employment agreement ("the Agreement") and therefore:

IT IS AGREED AS FOLLOWS:

1 Commencement date Agreement and Position

- 1.1 Effective July 15, 2017, the Initial Agreement will be replaced by this Agreement by which the Employee will perform the position of Chief Accounting Officer, Job grade 17, and undertakes to perform all the activities that can reasonably be assigned to him by or on behalf of the Employer and which are related to the Employer's business, to the best of his ability and in doing so, will comply with the instructions given to him by or on behalf of the Employer.
- 1.2 The Employer shall be entitled to assign other duties than the usual activities of the Employee, or to alter the position of the Employee if in the opinion of the Employer in reasonableness the business circumstances so require.
- 1.3 When the Agreement is entered into, the work will be performed at the office of the Employer at Paasheuvelweg 25a (1105 BP) Amsterdam. The Employer reserves the right to change the location where the work is performed after consultation with the Employee.
- 1.4 The normal working hours are 40 hours per week (1,0 FTE). The working hours are normally 8,0 hours a day and a 30 minutes lunch break.

2 Term and Termination Agreement

- 2.1 The Agreement has been entered into for an indefinite period of time.
-

- 2.2 The Agreement will in any event, without notice being required, terminate as of the first day of the month following the date the Employee reaches the State pension age (*AOW-gerechtigde leeftijd*).
- 2.3 The Agreement can be terminated by each of the Parties with due observance of the statutory notice period of 4 months for the Employer and 2 months for the Employee.
- 2.4 If the Agreement is terminated on the initiative of Employer, other than in the case of summary dismissal as referred to in article 7:677 of the Dutch Civil Code, long-term illness (article 7:669 section 3 under b Dutch Civil Code) or severely culpable acts or omissions by Employee as referred to in article 7:669 section 3 under e Dutch Civil Code, Employer shall grant the Employee severance pay equal to 100% of the annual base salary excluding 8 % holiday allowance (hereinafter: 'Severance pay') subject to deductions that are authorized by Employee and/or required by applicable laws and regulations. If and insofar Employee is entitled to the transition payment as referred to in article 7:673 of the Dutch Civil Code, this transition payment shall be deemed to be factored into the Severance pay. In the event of a termination under this clause the Employer shall provide 4 months' notice to the Employee. The Employer, in its sole discretion, may choose to put the Employee on garden leave at any time during the notice period. Garden leave will be considered equal to continuation of full time employment, but the Employee will be released from his working duties. In the event that the Employee is placed on garden leave, the amount of the severance pay under this clause 2.4 will be reduced by an amount equivalent to the salary during the Employee's garden leave.

Change of Control

- 2.5 Change of Control will be defined as follows: *"A situation in which more than 50% of the shares in the Employer will be acquired by a third party, and as a result whereof, the Employer becomes part of another group of companies, whether or not with the view of terminating the stock exchange listing of the Employer."*
- 2.6 In the event of a proposed Change of Control, the conditions as stated in Clause 2.7. through 2.11 of the Agreement shall apply instead of the provisions in Clause 2.4, provided that the Employee is actively working and performing services for the Employer throughout the full process leading up to the actual Change of Control ("the Closing Date").
- 2.7 The Employer cannot give notice of termination for reasons related to the Change of Control earlier than two months before the expected Closing Date and provided that the proposed Change of Control has been duly announced by means of a press release.
- 2.8 In case the Employer gives notice of termination for reasons related to the Change of Control, a notice period for the Employer of 12 months applies instead of the notice period of four months as mentioned in Clause 2.3 of the Agreement. The 12 months' notice period starts from the moment notice has been given. During the notice period the Employee is entitled to full salary and all other terms and conditions of employment will continue to apply, including blackout periods (no share trading).
- 2.9 Upon the Employee's written request the entitlement, as referred to in the last sentence of Clause 2.8. of this Agreement, can be converted to an one-time cash payout. This cash payout consists of the equivalent of the full salary the Employee is entitled to, excluding other terms and conditions, for the remaining part of the notice period. The Employee can file such a
-

request anytime throughout the notice period. At the time of such payout, the employment will be deemed terminated. If and insofar Employee is entitled to the transition payment as referred to in article 7:673 of the Dutch Civil Code, this transition payment shall be deemed to be factored into the cash payout.

- 2.10 During the notice period the Employer may choose to put the Employee on garden leave. Garden leave will be considered equal to continuation of full time employment, but the Employee will be released from his working duties. The Employee can be called for work during his garden leave without any extra compensation.
- 2.11 Clauses 2.5 through 2.11. of this Agreement are conditional upon fully performed employment by the Employee throughout the process of Change of Control until the Closing Date. Clauses 2.11. and 2.8. are not applicable if (i) the Employee gives notice prior to the Closing date, (ii) the Employee has not fully performed his duties in the period prior to the Closing date, and/or (iii) the Employee is offered employment by the acquiring entity (i.e. the new shareholder) or one of the group entities of the new shareholder, provided that the employment terms and conditions offered by the acquiring entity are at least equal to the terms and conditions of the Agreement, the Employee performs his services at the same location and the Employee remains employed by the new shareholder for at least 18 months after the Closing Date (not including the notice period).

3 Salary and holiday allowance

- 3.1 The Employee's annual salary will be EUR 200.000,00 gross, including 8% holiday allowance on the basis of a fulltime employment. The salary, excluding the holiday allowance, shall be paid in 12 equal instalments ultimately by the end of each calendar month. The Employee will work on a fulltime basis, 1,0 FTE and therefore the actual monthly salary is EUR 15.432,10. In case of part time employment, all earnings will be pro-rated.
- 3.2 Once per year, in the month May, the Employer shall pay to the Employee the holiday allowance of 8% of the annual gross salary earned with the Employer in the preceding calendar year. If the Agreement commences and/or terminates during the calendar year, and/ or the Employee works on a part-time basis, the holiday allowance will be paid out pro rata.
- 3.3 The Employee shall be eligible to a bonus payment amounting to a maximum of 35% of his annual gross salary. The Employee's eligibility to a bonus payments shall be dependent on the company guidelines and discretionary to the Management Board.
- 3.4 In July 2017 the Employee will be paid a spot on bonus of EUR 20.000,00 gross as appreciation for duties performed in the past 9 months.
- 3.5 Bonus payments, if any, are ultimately made in the month following the month in which the annual accounts of the Employer were adopted by the meeting of the shareholders. Bonus payments, if any, will not be taken into account for the calculation of any possible severance payment upon termination of the Agreement. Employee needs to be in service on date of bonus pay out.

4 Overtime

The Employee undertakes to work overtime at the request of the Employer. The Employer does not pay any compensation for overtime.

5 Expenses

- 5.1 The costs for travelling from home to office shall be compensated in accordance with the company policy.
- 5.2 To the extent that the Employer has given prior approval for business travels, the Employer shall reimburse reasonable travel and accommodation expenses relating to such business travel incurred by the Employee in the performance of his duties upon submission of all the relevant invoices and vouchers within 30 days following completion of the business travel.

6 Holidays

- 6.1 The Employee is entitled to 30 business days holiday per year or a pro rata portion thereof if the Agreement commences and/or terminates during the calendar year and/ or the Employee works part-time.
- 6.2 The statutory holiday days (20 days of the 30 per year on full time employment) shall be forfeited after 6 months after the end of the year in which the holiday days were accrued.
- 6.3 The Employer shall determine the commencement and the end of the holiday in consultation with the Employee. The Employee shall take his holidays in the period that the activities best allow this.

7 Illness

In the event of illness in the sense of section 7:629 Dutch Civil Code, the Employee must report sick to the Employer as soon as possible, but no later than 9 a.m. on the first day of illness. The Employee undertakes to comply with the rules related to reporting and inspection in the case of illness, as adopted from time to time by the Employer.

8 Insurance

The Employer will comply with the obligations under the Dutch Health Care Insurance Act.

9 Pension

The Employee shall be entitled to participate in the pension scheme of the Employer following company guidelines.

10 Confidentiality obligation

- 10.1 Both during the term of the Agreement and after the Agreement has been terminated for any reason whatsoever, the Employee shall not make any statements in any way whatsoever to anyone whomsoever (including other personnel of the Employer, unless these should be informed of anything in connection with the work they perform for the Employer), regarding matters, activities and interests of a confidential nature related to the business of the Employer and/or the Employer's affiliates, of which the Employee became aware within the scope of his work for the Employer and the confidential nature of which he is or should be aware. This includes, *inter alia*, information about the Employer's products, processes and services, including but not limited to, information relating to research, development, inventions, manufacture, purchasing, engineering, marketing, merchandising and selling.
-

10.2 For all oral and written publications by the Employee, which can or could harm the interests of the Employer, prior approval from the Employer has to be obtained. This approval shall only be refused on sincere grounds based on those interests.

10.3 All information exchanged via the Employer's email system is considered to be company's proprietary information and should be taken care of accordingly.

11 Documents

The Employee is prohibited from in any way having documents and/or correspondence and/or other information carriers and/or copies thereof in his possession that belong to the Employer and/or to the Employer's affiliates, with the exception of the extent to which and as long as required for the performance of his activities for the Employer. In any event, the Employee is required, even without any request being made to that end, to return such documents and/or correspondence and/or other information carriers and/or copies thereof to the Employer immediately upon the end of the Agreement, or in the event the Employee is on non-active duty for any reason whatsoever.

12 Ban on ancillary jobs

During the term of the Agreement, without the prior written consent of the Employer, the Employee shall not accept any paid work or time-consuming unpaid work at or for third parties and will refrain from doing business for his own account.

13 Non-competition and business relationship clause

13.1 Both during the term of the Agreement and for a period of one year after the Agreement has been terminated for any reason whatsoever, without the prior written consent of the Employer, the Employee shall not be engaged or involved or have any share in any manner whatsoever, directly or indirectly, whether on his own behalf or for third parties, in any enterprise which conducts activities in a field similar to or otherwise competing with that of the Employer and/or the Employer's affiliates, nor act, in any manner whatsoever, directly or indirectly, whether on his own behalf or for third parties, as an intermediary in relation to such activities.

13.2 Both during the term of the Agreement and for a period of one year after the Agreement has been terminated for any reason whatsoever, without the prior written consent of the Employer, the Employee shall not perform or have performed professional services in connection with any product or research or development or commercialization that competes with products, or research or development or commercialization of Employer, directly or indirectly, whether on his own behalf or for third parties, nor enter into contact, in that respect, directly or indirectly, whether on his own behalf or for third parties, with clients and/or relations of the Employer and/or the Employer's affiliates and/or purchasers of products and/or services of the Employer and/or the Employer's affiliates.

13.3 Clients and/or relations of the Employer and/or the Employer's affiliates such as set out in article 13.2 of this Agreement shall in all events mean relations of the Employer and/or the Employer's affiliates with which the Employer has or has had (business) contact in any manner whatsoever throughout the course of, or otherwise prior to the termination of, the Agreement.

13.4 Both during the term of the Agreement and for a period of one year after the Agreement has been terminated for any reason whatsoever, without the prior written consent of the Employer,

the Employee shall refrain from becoming engaged or involved in any manner whatsoever, directly or indirectly, whether on his own behalf or for third parties, in actively enticing away, taking (or causing to have taken) into employment, nor make use of, in any manner whatsoever, directly or indirectly, whether on his own behalf or for third parties, the type of work of employees or persons who in a period of one year prior to the termination of the Agreement of the Employee are or have been in the employment of the Employer and/or the Employer's affiliates.

- 13.5 Employee acknowledges and agrees to the adhere to this clause as the Employer has a serious business interest in binding the Employee to the non-competition and business relationship clause, due to the fact that (i) within the organization of the Employer competition-sensitive information as well as confidential information related to the Employer and its clients and relations, such as but not limited to products, or research or development or commercialization of Employer ("Sensitive Business Information") are available and (ii) in the position of **Chief Accounting Officer** the Employee has access to this Sensitive Business Information and/or will become aware of this Sensitive Business Information and/or will maintain (commercial) contacts with clients, suppliers, competitors etc. Given the aforesaid considerations (i) and (ii) in this clause, combined with the education and capacities of the Employee, the Employer has a well-founded fear that its business interest will be harmed substantially if the Employee performs competing activities as set forth in clauses 13.1 up to and including 13.5 of the Agreement within a period of 12 months after termination of the Agreement.

14 Intellectual and industrial property

- 14.1 The Employer is or will be considered to be, to the fullest extent allowed by law, the maker/producer/designer/breeder of all that which is made, created, improved, produced, designed, invented or discovered by the Employee during the course of this Agreement (the **Works**).
- 14.2 The Employee is obliged to fully and comprehensibly disclose all Works to the Employer in writing immediately after they are created or after the creation becomes known to the Employee, and in any case at the request of the Employer.
- 14.3 The Employee hereby transfers and assigns all his rights to and in connection with the Works to the Employer in advance.
- 14.4 The Employee is obliged, at first request of the Employer, to transfer and assign to the Employer all rights to and in connection with the Works that do not belong to the Employer by operation of law (*van rechtswege*), and that are not transferred to the Employer pursuant to article 14.3 of this Agreement. This concerns all rights, anywhere in the world, to and arising from or in connection with the Works. This obligation of the Employee remains in force even after the end of this Agreement.
- 14.5 The Employee agrees to perform, to the extent necessary and/or at the request of the Employer, such further acts as may be necessary or desirable to apply for, obtain and/or maintain protection for the Works, *inter alia* by means of the establishment of intellectual and industrial property rights. The Employee hereby grants permission and power of attorney to the Employer to the extent necessary to carry out every required act on behalf of the Employee to obtain protection for the Works, or to transfer the Works and any rights relating thereto, to
-

the Employer. The Employer will compensate the reasonable costs made in respect hereof, in so far as the payment that the Employee receives pursuant to article 3.1 of this Agreement cannot be considered as compensation for such costs. This obligation of the Employee remains in force even after the end of the Agreement.

- 14.6 The Employee acknowledges that the payment ex article 3.1 of this Agreement includes a reasonable compensation for any possible deprivation of any intellectual and industrial property rights. To the extent legally possible, the Employee hereby waives his right to any additional compensation with respect to the Works.

15 Gifts

In connection with the performance of his duties, the Employee is prohibited from accepting or stipulating, either directly or indirectly, any commission, reimbursement or payment, in whatever form, or gifts from third parties. The foregoing does not apply to standard promotional gifts having little monetary value.

16 Penalty clause

In the event the Employee acts in violation of any of the obligations under the articles 10 through 15 of this Agreement, the Employee shall, contrary to section 7:650 paragraphs 3, 4 and 5 Dutch Civil Code, without notice of default being required, forfeit to the Employer for each such violation, a penalty in the amount of EUR 10.000,00 as well as a penalty of EUR 1.000,00 for each day such violation has taken place and continues. Alternatively, the Employer will be entitled to claim full damages.

17 Transfer of an undertaking

The Employee shall remain under the obligation to adhere the set out in the articles 10 through 16 of this Agreement vis-à-vis the Employer, if the enterprise of the Employer or a part thereof is transferred to a third party within the meaning of section 7:662 and onwards Dutch Civil Code and this Agreement terminates before or at the time of such transfer, whereas in the event of continuation of the Agreement the Employee would have entered the employment of the acquirer by operation of law.

18 Other arrangements

Subject to the provisions in this Agreement, the arrangements related to employment conditions adopted by the Employer from time to time, as laid down in the Employee Handbook are applicable. A copy of these arrangements has been provided to the Employee. By signing this agreement, the Employee acknowledges to have received and understood the Employee Handbook and the Insider Trading Policy.

19 Employment costs regulation

The conditions of employment costs regulation determined by the Employer apply. In this context, the Employer reserves the right at its sole discretion to modify certain fringe benefits, without any compensation in return.

20 Amendment clause

- 20.1 The Employer reserves the right to unilaterally amend the Agreement and the arrangements referred to in article 18 of this Agreement if it has such a serious interest in that respect entailing that the interests of the Employee must yield to that in accordance with standards of reasonableness and fairness.
- 20.2 The Employer reserves the right to unilaterally amend the Agreement and the arrangements referred to in article 18 of this Agreement in the event of a relevant amendment of the law.

21 Applicable law, no collective labour agreement

- 21.1 This Agreement is governed by Dutch law.
- 21.2 The Agreement is not subject to any collective labour agreement.

THIS AGREEMENT has been entered into on the date stated at the beginning of this Agreement

uniQure biopharma B.V.

uniQure biopharma B.V.

/s/ Matthew Kapusta

By: Matthew Kapusta
Title: CEO

By: _____
Title: _____

Employee

/s/ Christian Klemm

By: Christian Klemt

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; and
 - (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; and
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
August 8, 2017

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; and
 - (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; and
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
August 8, 2017

**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 June 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
August 8, 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.
