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, 2020

OR

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

For the transition period from _____ to _____

Commission file number: 001-36294

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

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

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

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

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

Title of each class:	**Trading Symbol(s)**	**Name of each exchange on which registered**
Ordinary Shares	QURE	The Nasdaq Global Select Market

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 every Interactive Data File required to be submitted 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 such files). Yes ☒ No ☐.

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

Large accelerated filer ☒	Accelerated filer ☐
Non-accelerated filer ☐	Smaller reporting company ☐
	Emerging growth company ☐

If an emerging growth company, indicate by check mark if the registrant has elected not to use the extended transition period for complying with any new or revised financial accounting standards provided pursuant to Section 13 (a) of the Exchange Act ☐

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 July 28, 2020, the registrant had 44,446,468 ordinary shares, par value €0.05, outstanding.

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SPECIAL CAUTIONARY NOTICE REGARDING FORWARD-LOOKING STATEMENTS

This Quarterly Report on Form 10-Q contains "forward-looking statements" as defined under federal securities laws. Forward-looking statements are based on our current expectations of future events and many of these statements can be identified using terminology such as "believes," "expects," "anticipates," "plans," "may," "will," "projects," "continues," "estimates," "potential," "opportunity" and similar expressions. These forward-looking statements, which include statements related to the COVID-19 coronavirus pandemic, 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 are only predictions based on management's current views and assumptions and involve risks and uncertainties, and actual results could 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 Quarterly Report on Form 10-Q, 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 SEC on March 2, 2020, 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 forward-looking 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, 2020, and in our Annual Report on Form 10-K for the year ended December 31, 2019, 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 CONSOLIDATED BALANCE SHEETS

	June 30, 2020	December 31, 2019
	(in thousands, except share and per share amounts)	
Current assets		
Cash and cash equivalents	$ 314,265	$ 377,793
Accounts receivable and accrued income from related party	222	947
Prepaid expenses	4,082	4,718
Other current assets	1,066	748
Total current assets	**319,635**	**384,206**
Non-current assets		
Property, plant and equipment, net of accumulated depreciation of $31.3 million as of June 30, 2020 and $28.6 million as of December 31, 2019, respectively	29,301	28,771
Operating lease right-of-use assets	26,139	26,797
Intangible assets, net	7,087	5,427
Goodwill	496	496
Restricted cash	2,683	2,933
Total non-current assets	**65,706**	**64,424**
Total assets	**$ 385,341**	**$ 448,630**
Current liabilities		
Accounts payable	$ 4,942	$ 5,681
Accrued expenses and other current liabilities	13,250	12,457
Current portion of operating lease liabilities	5,495	5,865
Current portion of deferred revenue	6,153	7,627
Total current liabilities	**29,840**	**31,630**
Non-current liabilities		
Long-term debt	35,373	36,062
Operating lease liabilities, net of current portion	30,279	31,133
Deferred revenue, net of current portion	23,048	23,138
Derivative financial instruments related party	832	3,075
Other non-current liabilities	464	534
Total non-current liabilities	**89,996**	**93,942**
Total liabilities	**119,836**	**125,572**
Commitments and contingencies		
Shareholders' equity		
Ordinary shares, €0.05 par value: 60,000,000 shares authorized at June 30, 2020 and December 31, 2019 and 44,444,405 and 43,711,954 ordinary shares issued and outstanding at June 30, 2020 and December 31, 2019, respectively	2,692	2,651
Additional paid-in-capital	1,000,389	986,803
Accumulated other comprehensive loss	(7,319)	(6,689)
Accumulated deficit	(730,257)	(659,707)
Total shareholders' equity	**265,505**	**323,058**
Total liabilities and shareholders' equity	**$ 385,341**	**$ 448,630**

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

uniQure N.V.

UNAUDITED CONSOLIDATED STATEMENTS OF OPERATIONS AND COMPREHENSIVE LOSS

	Three months ended June 30,		Six months ended June 30,	
	2020	2019	2020	2019
	(in thousands, except share and per share amounts)		(in thousands, except share and per share amounts)	
License revenues from related party	1,530	2,108	1,577	2,665
Collaboration revenues from related party	5	366	62	945
Total revenues	**1,535**	**2,474**	**1,639**	**3,610**
Operating expenses:				
Research and development expenses	(28,401)	(24,154)	(54,414)	(44,691)
Selling, general and administrative expenses	(11,511)	(7,870)	(20,583)	(15,937)
Total operating expenses	**(39,912)**	**(32,024)**	**(74,997)**	**(60,628)**
Other income	669	566	1,526	879
Other expense	(500)	(347)	(839)	(696)
Loss from operations	**(38,208)**	**(29,331)**	**(72,671)**	**(56,835)**
Interest income	81	728	903	1,170
Interest expense	(970)	(937)	(1,945)	(1,894)
Foreign currency (losses) / gains, net	(3,645)	(1,252)	957	1,022
Other non-operating gains / (losses), net	191	(607)	2,206	(2,634)
Net loss	**$ (42,551)**	**$ (31,399)**	**$ (70,550)**	**$ (59,171)**
Other comprehensive income / (loss):				
Foreign currency translation adjustments	4,647	1,209	(630)	(1,259)
Total comprehensive loss	**$ (37,904)**	**$ (30,190)**	**$ (71,180)**	**$ (60,430)**
Basic and diluted net loss per ordinary share	$ (0.96)	$ (0.83)	$ (1.59)	$ (1.57)
Weighted average shares used in computing basic and diluted net loss per ordinary share	44,387,463	37,824,928	44,333,460	37,750,961

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

uniQure N.V.

UNAUDITED CONSOLIDATED STATEMENTS OF SHAREHOLDERS' EQUITY
FOR THE THREE-MONTH PERIOD ENDED JUNE 30

	Ordinary shares		Additional paid-in capital	Accumulated other comprehensive (loss)/income	Accumulated deficit	Total shareholders' equity
	No. of shares	Amount				
	(in thousands, except share and per share amounts)					
Balance at March 31, 2019	**37,763,842**	**$ 2,322**	**$ 727,795**	**$ (9,727)**	**$ (563,278)**	**$ 157,112**
Loss for the period	—	—	—	—	(31,399)	(31,399)
Other comprehensive income	—	—	—	1,209	—	1,209
Hercules warrants exercise	—	—	—	—	—	-
Exercise of share options	52,847	4	454	—	—	458
Restricted and performance share units distributed during the period	21,400	1	(1)	—	—	—
Share-based compensation expense	—	—	4,592	—	—	4,592
Issuance of ordinary shares relating to employee stock purchase plan	1,744	—	84	—	—	84
Balance at June 30, 2019	**37,839,833**	**$ 2,327**	**$ 732,924**	**$ (8,518)**	**$ (594,677)**	**$ 132,056**
Balance at March 31, 2020	**44,299,596**	**$ 2,683**	**$ 992,136**	**$ (11,966)**	**$ (687,706)**	**$ 295,147**
Loss for the period	—	—	—	—	(42,551)	(42,551)
Other comprehensive income	—	—	—	4,647	—	4,647
Exercise of share options	139,178	9	2,461	—	—	2,470
Restricted and performance share units distributed during the period	4,427	—	—	—	—	—
Share-based compensation expense	—	—	5,723	—	—	5,723
Issuance of ordinary shares relating to employee stock purchase plan	1,204	—	69	—	—	69
Balance at June 30, 2020	**44,444,405**	**$ 2,692**	**$ 1,000,389**	**$ (7,319)**	**$ (730,257)**	**$ 265,505**

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

uniQure N.V.

UNAUDITED CONSOLIDATED STATEMENTS OF SHAREHOLDERS' EQUITY
FOR THE SIX-MONTH PERIOD ENDED JUNE 30

	Ordinary shares		Additional paid-in capital	Accumulated other comprehensive (loss)/income	Accumulated deficit	Total shareholders' equity
	No. of shares	Amount				
	(in thousands, except share and per share amounts)					
Balance at December 31, 2018	**37,351,653**	**$ 2,299**	**$ 720,072**	**$ (7,259)**	**$ (535,506)**	**$ 179,606**
Loss for the period	—	—	—	—	(59,171)	(59,171)
Other comprehensive loss	—	—	—	(1,259)	—	(1,259)
Hercules warrants exercise	37,175	2	1,271	—	—	1,273
Exercise of share options	236,654	14	2,542	—	—	2,556
Restricted and performance share units distributed during the period	209,481	12	(12)	—	—	—
Share-based compensation expense	—	—	8,886	—	—	8,886
Issuance of ordinary shares relating to employee stock purchase plan	4,870	—	165	—	—	165
Balance at June 30, 2019	**37,839,833**	**$ 2,327**	**$ 732,924**	**$ (8,518)**	**$ (594,677)**	**$ 132,056**
Balance at December 31, 2019	**43,711,954**	**$ 2,651**	**$ 986,803**	**$ (6,689)**	**$ (659,707)**	**$ 323,058**
Loss for the period	—	—	—	—	(70,550)	(70,550)
Other comprehensive loss	—	—	—	(630)	—	(630)
Exercise of share options	203,940	12	3,390	—	—	3,402
Restricted and performance share units distributed during the period	525,506	29	(29)	—	—	—
Share-based compensation expense	—	—	10,078	—	—	10,078
Issuance of ordinary shares relating to employee stock purchase plan	3,005	—	147	—	—	147
Balance at June 30, 2020	**44,444,405**	**$ 2,692**	**$ 1,000,389**	**$ (7,319)**	**$ (730,257)**	**$ 265,505**

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

uniQure N.V.

UNAUDITED CONSOLIDATED STATEMENTS OF CASH FLOWS

	Six months ended June 30,	
	2020	2019
	(in thousands)	
Cash flows from operating activities		
Net loss	$ (70,550)	$ (59,171)
Adjustments to reconcile net loss to net cash used in operating activities:		
Depreciation and amortization	3,505	3,217
Share-based compensation expense	10,078	8,886
Change in fair value of derivative financial instruments	(2,206)	2,634
Unrealized foreign exchange gains	(908)	(386)
Changes in operating assets and liabilities:		
Accounts receivable and accrued income, prepaid expenses and other current assets	1,050	(2,779)
Accounts payable	(981)	283
Accrued expenses, other liabilities and operating leases	(1,332)	(658)
Deferred revenue	(1,600)	(2,683)
Net cash used in operating activities	(62,944)	(50,657)
Cash flows from investing activities		
Purchases of intangible assets	(2,214)	(996)
Purchases of property, plant and equipment	(2,392)	(1,432)
Net cash used in investing activities	(4,606)	(2,428)
Cash flows from financing activities		
Proceeds from issuance of shares related to employee stock option and purchase plans	3,549	2,721
Proceeds from exercise of warrants	-	500
Net cash generated from financing activities	3,549	3,221
Currency effect on cash, cash equivalents and restricted cash	223	(443)
Net decrease in cash, cash equivalents and restricted cash	(63,778)	(50,307)
Cash, cash equivalents and restricted cash at beginning of period	380,726	237,342
Cash, cash equivalents and restricted cash at the end of period	**$ 316,948**	**$ 187,035**
Cash and cash equivalents	$ 314,265	$ 184,095
Restricted cash related to leasehold and other deposits	2,683	2,940
Total cash, cash equivalents and restricted cash	**$ 316,948**	**$ 187,035**
Supplemental cash flow disclosures:		
Cash paid for interest	$ (2,545)	$ (1,544)
Non-cash increases in accounts payables and accrued expenses and other current liabilities related to purchases of intangible assets and property, plant and equipment	$ 1,021	$ 473

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

1 General business information

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

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

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

2 Summary of significant accounting policies

2.1 Basis of preparation

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

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

2.2 Unaudited interim financial information

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

Certain information and footnote disclosures normally included in annual financial statements prepared in accordance with U.S. GAAP have been omitted. The results of operations for the six months ended June 30, 2020, are not necessarily indicative of the results to be expected for the full year ending December 31, 2020 or for any other future year or interim period. The accompanying financial statements should be read in conjunction with the audited financial statements and the related notes thereto included in the Company's Annual Report on Form 10-K for the year ended December 31, 2019, filed with the SEC on March 2, 2020.

2.3 Use of estimates

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

2.4 Accounting policies

The principal accounting policies applied in the preparation of these unaudited consolidated financial statements are described in the Company's audited financial statements as of and for the year ended December 31, 2019, and the notes thereto, which are included in the Company's Annual Report on Form 10-K for the year ended December 31, 2019, filed with the SEC on March 2, 2020. There have been no material changes in the Company's significant accounting policies during the six months ended June 30, 2020.

2.5 Recent accounting pronouncements

There have been no new accounting pronouncements or changes to accounting pronouncements during the six months ended June 30, 2020, as compared to the recent accounting pronouncements described in Note 2.3.23 of the Company's Annual Report on Form 10-K for the year ended December 31, 2019, which could be expected to materially impact the Company's unaudited consolidated financial statements. The Company has adopted ASU 2018-13, Fair Value Measurement (Topic 820) and additional disclosures related to significant unobservable inputs have been included within footnote 4 Fair value measurement.

3 Collaboration arrangements and concentration of credit risk

CSL Behring collaboration

On June 24, 2020, uniQure biopharma B.V., a wholly-owned subsidiary of uniQure N.V., entered into a commercialization and license agreement (the "CSL Behring Agreement") with CSL Behring LLC ("CSL Behring") providing CSL Behring exclusive global rights to etranacogene dezaparvovec, the Company's investigational gene therapy for patients with hemophilia B (the "Product").

Under the terms of the CSL Behring Agreement, the Company will receive a $450 million upfront cash payment upon the closing of the CSL Behring Agreement and be eligible to receive up to $1.6 billion in payments based on regulatory and commercial milestones. The Company will also be eligible to receive tiered double-digit royalties in a range of up to a low-twenties percent of net sales of the Product based on sales thresholds.

Pursuant to the CSL Behring Agreement, the Company will be responsible for the completion of the HOPE-B clinical trial, manufacturing process validation, and the manufacturing supply of Product until such time that these capabilities may be transferred to CSL Behring or its designated contract manufacturing organization. Pursuant to a development and commercial supply agreement, the Company will supply Product to CSL Behring at an agreed-upon price per the contract. The Company and CSL Behring executed the development and commercial supply agreement simultaneously with the CSL Behring Agreement. Certain provisions will not become effective until after the Company receives regulatory approval to close the transaction. Clinical development and regulatory activities performed by the Company pursuant to the CSL Behring Agreement will be reimbursed by CSL Behring. CSL Behring will be responsible for global regulatory submissions and commercialization requirements for Product.

Closing of the CSL Behring Agreement is contingent on the successful completion of reviews under antitrust laws in the United States, Australia, and the United Kingdom, which has not occurred to date. Closing of the transaction is dependent on the timing, extent, and result of the regulatory review process. The Company does not believe that the consummation of the transaction will result in a violation of any applicable antitrust laws. However, there can be no assurance that a challenge on antitrust grounds will not be made, or if such a challenge is made, what the result would be. In accordance with its existing license and other agreements the Company is contractually required to pay in total a low to high single digit percentage of any upfront payment to its licensors and financial advisor ("License Fees").

As of June 30, 2020, the Company concluded it has no enforceable right to receive any of the upfront payment, the regulatory and sales milestone payments or the royalties (together "CSL License Revenue") that it may receive in accordance with the CSL Behring Agreement, as all payments are contingent upon the successful completion of reviews under antitrust laws in the United States, Australia and the United Kingdom. Therefore, the Company determined it will not recognize any revenue in relation to the CSL License Revenue, in accordance with ASC 606.

The Company determined that in accordance with Dutch taw law it will recognize the CSL Behring License Revenue as well as the License Fees as taxable results as of the date on which the Company is contractually entitled to receive (or obligated to make) a payment under the CSL Behring Agreement. The Company expects to continue to incur taxable losses in the Netherlands except for the period in which it receives the $450 million upfront payment. In the event that the Company recognizes the $450 million upfront payment in 2020, such payment will be subject to Dutch corporate income tax at a rate of 25.0%. Any CSL License Revenue including the upfront payment that the Company recognizes thereafter will be subject to Dutch corporate income tax at a rate of 21.7%. However, the Company does not expect it will be required to pay any income taxes in the period in which it recognizes the $450 million upfront payment as taxable revenue as such payment is not expected to exceed the net operating losses the Company has carried forward in the Netherlands. The Company has historically recorded a full valuation allowance against its net deferred tax assets.

Future taxable income or losses of the Company and a potential reversal of the valuation allowance will be impacted by a variety of factors, of which some are outside of the Company's control. Such factors include the outcome and timing of the reviews under antitrust laws in the United States, Australia, and the United Kingdom, the change in the Dutch corporate income tax rate in 2021, additional net operating losses the Company may generate in relation to the CSL Behring Agreement, and the amount of License Fees the Company is required to pay.

The Company recognizes deferred tax assets to the extent that it determines that these assets are more likely than not to be realized. In making such a determination, the Company weighed all available positive and negative evidence, including future income projections from the CSL Behring Agreement, and concluded that it is more likely than not that the deferred tax assets will not be realized. Accordingly, the Company continued to record a full valuation allowance as of June 30, 2020.

Bristol-Myers Squibb collaboration

In May 2015, the Company entered into a collaboration and license agreement (the "BMS CLA") and various related agreements with Bristol-Myers Squibb Company ("BMS") that provide BMS with exclusive access to the Company's gene therapy technology platform for the research, development and commercialization of therapeutics aimed at multiple targets in cardiovascular and other diseases ("Collaboration Targets"). The initial four-year research term under the collaboration terminated on May 21, 2019. During the initial research term of the BMS CLA, the Company supported BMS in discovery, non-clinical, analytical and process development efforts in respect of the Collaboration Targets. For any Collaboration Targets that may be advanced, the Company will be responsible for manufacturing of clinical and commercial supplies using the Company's vector technologies and industrial, proprietary insect-cell based manufacturing platform. BMS reimbursed the Company for all its research and development costs in support of the collaboration during the initial research term, and will lead development, regulatory and commercial activities for any Collaboration Targets that may be advanced. The BMS CLA provides that the Company and BMS may collaborate on up to ten Collaboration Targets in total. As of June 30, 2020, BMS has designated a total of four Collaboration Targets. In February 2019, BMS requested a one-year extension of the research term. In April 2019, following an assessment of the progress of this collaboration and the Company's expanding proprietary programs, the Company notified BMS that the Company did not intend to agree to an extension of the research term but rather preferred to restructure or amend the collaboration to reduce or eliminate certain of the Company's obligations under it.

The Company has agreed to certain restrictions on its ability to work independently of the collaboration, either directly or indirectly through any affiliate or third party, on certain programs that would be competitive with the collaboration programs. The Company is currently in discussions with BMS potentially to amend the BMS CLA and other related agreements following the expiration of the research term. It is currently uncertain whether a change to the BMS CLA will be agreed and, if agreed, what the specific terms of any such change may be. As a consequence, the Company has not taken into account the impact of such change, if any, on the timing of recognition of the prepaid License Revenue if and when the BMS CLA and other related agreements have been restructured or amended. The final resolution of these discussions may or may not result in material changes to the Company's collaboration with BMS. The Company agreed, subject to certain conditions, to continue providing limited support of the pre-clinical Collaboration Targets, and any related costs will be reimbursed by BMS during these discussions.

The Company evaluated the BMS CLA and determined that its performance obligations are as follows:

(i) Providing access to its technology and know-how in the field of gene therapy as well as actively contributing to the target selection, the collaboration as a whole, the development during the target selection, the pre-clinical and the clinical phase through participating in joint steering committee and other governing bodies ("License Revenue");

(ii) Providing pre-clinical Collaboration Target specific, non-clinical, analytical and process development services during the initial research term, which ended on May 21, 2019 ("Collaboration Revenue"); and

(iii) Providing clinical and commercial manufacturing services for Collaboration Targets ("Manufacturing Revenue"). To date the Company has not generated any Manufacturing Revenue.

Amounts owed by BMS in relation the collaboration services are as follows:

	June 30, 2020	December 31, 2019
	(in thousands)	
Bristol Myers Squibb	$ 222	$ 947
Total	**$ 222**	**$ 947**

License Revenue

The Company recognized $1.5 million and $1.6 million of License Revenue for the three and six months ended June 30, 2020, respectively, compared to $2.1 million and $2.7 million during the same periods in 2019 in relation to a $60.1 million upfront payment recorded on May 21, 2015, as well as $15.0 million received in relation to the designation of the second, third and fourth Collaboration Targets in August 2015 (together "Consideration").

The Company would be entitled to an aggregate $16.5 million in target designation payments upon the selection of the fifth through tenth Collaboration Targets. The Company would also be eligible to receive research, development and regulatory milestone payments of up to $254.0 million for a lead Collaboration Target and up to $217.0 million for each of the other selected Collaboration Targets, if defined milestones are achieved. The Company would include the variable consideration related to the selection of the fifth to tenth Collaboration Target, or any of the milestones, in the transaction price once it is considered probable that including these payments in the transaction price would not result in the reversal of cumulative revenue recognized. The Company would recognize significant amounts of License Revenue for services performed in prior periods if and when the Company considers this probable. Due to the significant uncertainty surrounding the development of gene-therapy product candidates and the dependence on BMS's performance and decisions, the Company does not currently consider this probable.

Additionally, the Company is eligible to receive net sales-based milestone payments and tiered mid-single to low double-digit royalties on product sales. 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 the first commercial sale if there is no such exclusivity. These revenues will be recognized when performance obligations are satisfied.

The Company recognizes License Revenue over the expected performance period based on its measure of progress towards the completion of certain activities related to its services. The Company determines such progress by comparing activities performed at the end of each reporting period with total activities expected to be performed. The Company estimates total expected activities using a number of unobservable inputs, such as the probability of BMS designating additional targets, the probability of successfully completing each phase and estimated time required to provide services during the various development stages. If available, the Company uses product candidate-specific research and development plans. Alternatively, the Company assumes that completion of the pre-clinical phase requires an average of four years and that clinical development and commercial launch on average require 8.5 years.

4 Fair value measurement

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

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

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

The carrying amount of cash and cash equivalents, accrued income from the related party, 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, 2020, and December 31, 2019:

	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, 2019					
Assets:					
Cash, cash equivalents and restricted cash	$ 380,726	$ —	$ —	$ 380,726	Cash and cash equivalents; restricted cash
Total assets	$ 380,726	$ —	$ —	$ 380,726	
Liabilities:					
Derivative financial instruments - related party	—	—	3,075	3,075	
Total liabilities	$ —	$ —	$ 3,075	$ 3,075	
At June 30, 2020					
Assets:					
Cash, cash equivalents and restricted cash	$ 316,948	$ —	$ —	$ 316,948	Cash and cash equivalents; restricted cash
Total assets	$ 316,948	$ —	$ —	$ 316,948	
Liabilities:					
Derivative financial instruments - related party	$ —	$ —	$ 832	$ 832	
Total liabilities	$ —	$ —	$ 832	$ 832	

Changes in Level 3 items during the six months ended June 30, 2020, are as follows:

	Derivative financial instruments
	(in thousands)
Balance at December 31, 2019	**$ 3,075**
Net gains recognized in profit or loss	(2,206)
Currency translation effects	(37)
Balance at June 30, 2020	**$ 832**

BMS warrants

The Company issued derivative financial instruments related to its collaboration with BMS. The fair value of the BMS derivative financial instruments ("BMS warrants") as of June 30, 2020, was $0.8 million compared to a fair value of $3.1 million as of December 31, 2019. Changes in the fair value of the BMS warrants are primarily impacted by changes in the Company's share price, whereby a decrease in share price generally results in a decrease of the fair value. In addition, the Company revised certain unobservable inputs as well as valuation techniques in March 2020 which resulted in a further decrease in fair value of $0.7 million when compared to December 31, 2019. These BMS warrants are described in more detail below.

The Company granted BMS two warrants:

- A warrant allowing BMS to purchase a specific number of the Company's unregistered ordinary shares such that its ownership will equal 14.9% immediately after such purchase ("1st warrant"). The 1st 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 BMS CLA) associated with the first six new targets (a total of seven Collaboration Targets); and (ii) the date on which BMS designates the sixth new target (the seventh Collaboration Target).
- A warrant allowing BMS to purchase a specific number of the Company's unregistered ordinary shares such that its ownership will equal 19.9% immediately after such purchase ("2nd warrant" and together with the 1st warrant, the "warrants"). The 2nd warrant can be exercised on the later of (i) the date on which the Company receives from BMS the Target Designation Fees associated with the first nine new targets (a total of ten Collaboration Targets); and (ii) the date on which BMS designates the ninth new target (the tenth Collaboration Target).

Pursuant to the terms of the BMS CLA, the exercise price in respect of each warrant is equal to the greater of (i) the product of (A) $33.84 and (B) a compounded annual growth rate of 10% (or approximately $55.07 as of June 30, 2020) and (ii) the product of (A) 1.10 and (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.

As of June 30, 2020, BMS had designated a total of four Collaboration Targets, and as such, the warrants were not exercisable. The Company estimated the exercise of warrants to occur within four and five years after the balance sheet date.

The significant unobservable inputs that the Company uses to develop Level 3 fair value measurements include the number of ordinary shares outstanding at the time of BMS's warrant exercises. The number of such ordinary shares outstanding at the time of exercise determines the number of unregistered ordinary shares to be issued in connection with the BMS warrants.

The warrants can only be exercised following the occurrence of events contractually defined in the warrant agreements. The probability of the occurrence of these events represent another significant unobservable input used in the calculation of the fair value of the warrants. The Company estimates that the probability of the occurrence of events allowing BMS to exercise the 1st warrant is within a range of 0% to 44% and between 0% to 11% with respect to the 2nd warrant. The arithmetic averages related to these ranges are 24% and 5% for the 1st and 2nd warrant, respectively.

5 Right-of-use assets and lease liabilities

The Company's most significant leases relate to office and laboratory space under the following operating lease agreements:

Lexington, Massachusetts / United States

In July 2013, the Company entered into a lease for a facility in Lexington, Massachusetts, United States. In November 2018, the term was expanded by five years from 2024 to June 2029. The lease is renewable for two subsequent five-year terms. Additionally, the lease was expanded to include an additional 30,655 square feet within the same facility and for the same term. The lease of the expansion space commenced on June 1, 2019.

Amsterdam / The Netherlands

In March 2016, the Company entered into a 16-year lease for a facility in Amsterdam, the Netherlands, and amended this agreement in June 2016. The lease for this facility terminates in February 2032, with an option to extend in increments of five-year periods. The lease contract includes variable lease payments related to annual increases in payments based on a consumer price index.

On December 1, 2017, the Company entered into an agreement to sub-lease three of the seven floors of its Amsterdam facility for a ten-year term ending on December 31, 2027, with an option for the sub-lessee to extend until December 31, 2031. In February 2020, the Company amended the agreement to sub-lease to take back one of the three floors effective March 1, 2020. The fixed lease payments to be received during the remaining term under the agreement to sub-lease amount to $6.4 million (EUR 5.7 million) as of June 30, 2020.

The table below presents the components of the Company's lease costs for the periods indicated:

	Three months ended June 30,		Six months ended June 30,	
	2020	2019	2020	2019
	(in thousands)		(in thousands)	
Operating lease cost	$ 1,289	$ 1,022	$ 2,579	$ 1,919
Variable lease cost	153	101	298	182
Sublease income	(209)	(264)	(457)	(532)
Total lease cost	**$ 1,233**	**$ 859**	**$ 2,420**	**$ 1,569**

The table below presents the lease-related assets and liabilities recorded on the Consolidated balance sheets for the periods indicated.

	June 30, 2020	December 31, 2019
	(in thousands)	
Assets		
Operating lease right-of-use assets	**$ 26,139**	**26,797**
Liabilities		
Current		
Current operating lease liabilities	5,495	5,865
Non-current		
Non-current operating lease liabilities	30,279	31,133
Total lease liabilities	**$ 35,774**	**36,998**

Other information

The weighted-average remaining lease term as of June 30, 2020 is 9.8 years, compared to 10.3 years as of December 31, 2019 and the weighted-average discount rate as of June 30, 2020 is 11.33%, compared to 11.33% as of December 31, 2019. The Company derived the weighted-average discount rate, adjusted for differences such as in the term and payment patterns, from the Company's loan from Hercules Capital which was refinanced immediately prior to the January 1, 2019 adoption date in December 2018.

Cash paid for amounts included in the measurement of lease liabilities for the periods indicated below was as follows:

	Three months ended June 30,		Six months ended June 30,	
	2020	2019	2020	2019
	(in thousands)		(in thousands)	
Operating cash flows for operating leases	$ 1,731	$ 603	$ 3,155	$ 2,016

The Company did not obtain any right-of-use assets in exchange for lease obligations in the three and six months ended June 30, 2020. Besides the initial recognition of operating right-of-use assets of $19.0 million upon adoption of the new lease standards on January 1, 2019, the Company obtained $8.6 million of additional right-of-use assets in exchange for lease obligations in the three and six months ended June 30, 2019.

Undiscounted cash flows

The table below reconciles the undiscounted cash flows as of June 30, 2020, for each of the first five years and the total of the remaining years to the operating lease liabilities recorded on the Consolidated balance sheet as of June 30, 2020.

	Lexington	Amsterdam[1)]	Other[1)]	Total
		(in thousands)		
2020 (six months remaining)	$ 1,697	$ 947	$ 61	$ 2,705
2021	3,455	1,894	141	5,490
2022	3,552	1,894	—	5,446
2023	3,650	1,894	—	5,544
2024	4,146	1,894	—	6,040
Thereafter	20,745	13,100	—	33,845
Total lease payments	$ 37,245	$ 21,623	$ 202	$ 59,070
Less: amount of lease payments representing interest payments	(13,790)	(9,498)	(8)	(23,296)
Present value of lease payments	23,455	12,125	194	35,774
Less: current operating lease liabilities	(3,407)	(1,894)	(194)	(5,495)
Non-current operating lease liabilities	**$ 20,048**	**$ 10,231**	**$ —**	**$ 30,279**

([1)] Payments are due in EUR and have been translated at the foreign exchange rate as of June 30, 2020, of $1.12/€1.00).

6 Accrued expenses and other current liabilities

Accrued expenses and other current liabilities include the following items:

	June 30, 2020	December 31, 2019
	(in thousands)	
Accruals for services provided by vendors-not yet billed	$ 6,629	$ 5,425
Personnel related accruals and liabilities	6,621	7,032
Total	**$ 13,250**	**$ 12,457**

7 Long-term debt

On June 14, 2013, the Company entered into a venture debt loan facility with Hercules Growth Capital, Inc. ("Hercules"), which was amended and restated on June 26, 2014, and again on May 6, 2016 ("2016 Amended Facility"). On December 6, 2018, the Company signed an amendment to the Second Amended and Restated Loan and Security Agreement that both refinanced the then-existing $20 million 2016 Amended Facility and provided an additional unconditional commitment of $15 million as well as a conditional commitment of $15 million that expired on June 30, 2020 (the "2018 Amended Facility"). At signing of the 2018 Amended Facility, the Company drew down an additional $15 million for a total of $35 million outstanding. The 2018 Amended Facility extended the loan's maturity date from May 1, 2020 until June 1, 2023. The interest-only period was initially extended from November 2018 to January 1, 2021 and was further extended to January 1, 2022 as a result of meeting the provision in the 2018 Amended Facility of raising more than $90.0 million in equity financing in September 2019. The Company is required to repay the facility in equal monthly installments of principal and interest between the end of the interest-only period and the maturity date. The interest rate continues to be adjustable and is the greater of (i) 8.85% and (ii) 8.85% plus the prime rate less 5.50% per annum.

Under the 2018 Amended Facility, the Company paid a facility fee of 0.50% of the $35 million outstanding as of signing and owes a back-end fee of 4.95% of the outstanding debt. In addition, in May 2020 the Company paid a back-end fee of $1.0 million in relation to the 2016 Amended Facility.

The amortized cost (including interest due presented as part of accrued expenses and other current liabilities) of the 2018 Amended Facility was $35.6 million as of June 30, 2020, compared to $36.3 million as of December 31, 2019, 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, 2020, was $0.7 million and $0.0 million compared to a foreign currency gain of $0.5 million during the three months ended June 30, 2019, and a foreign currency loss of $0.2 million during the six months ended June 30, 2019.

Interest expense associated with the 2018 Amended Facility during the three and six months ended June 30, 2020 was $0.9 million and $1.8 million, respectively, compared to $0.9 million and $1.9 million during the same periods in 2019.

As a covenant in the 2018 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 (i) 65% of the outstanding balance of principal due or (ii) 100% of worldwide cash and cash equivalents. This restriction on cash and cash equivalents only relates to the location of the cash and cash equivalents, and such cash and cash equivalents can be used at the discretion of the Company. In combination with other covenants, the 2018 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 directly or indirectly pledging its total assets of $385.3 million with the exception of $110.0 million of cash and cash equivalents and other current assets held by uniQure N.V.

The 2018 Amended Facility contains provisions that include the occurrence of a material adverse effect, as defined therein, which would entitle Hercules to declare all principal, interest and other amounts owed by the Company immediately due and payable. As of June 30, 2020, the Company was in compliance with all covenants and provisions.

8 Share-based compensation

The Company's share-based compensation plans include the 2014 Amended and Restated Share Option Plan (the "2014 Plan") and inducement grants under Rule 5653(c)(4) of the Nasdaq Global Select Market with terms similar to the 2014 Plan (together the "2014 Plans"). At the annual general meeting of shareholders in June 2018, the Company's shareholders approved amendments of the 2014 Plan, increasing the shares authorized for issuance by 3,000,000 to a total of 8,601,471.

a) 2014 Plans

Share-based compensation expense recognized by classification included in the Consolidated statements of operations and comprehensive loss in relation to the 2014 Plans for the periods indicated below was as follows:

	Three months ended June 30,		Six months ended June 30,	
	2020	2019	2020	2019
	(in thousands)		(in thousands)	
Research and development	$ 2,889	$ 2,119	$ 5,271	$ 4,099
Selling, general and administrative	2,825	2,457	4,783	4,755
Total	**$ 5,714**	**$ 4,576**	**$ 10,054**	**$ 8,854**

Share-based compensation expense recognized by award type for the periods indicated below was as follows:

	Three months ended June 30,		Six months ended June 30,	
	2020	2019	2020	2019
	(in thousands)		(in thousands)	
Award type				
Share options	$ 2,746	$ 2,004	$ 4,954	$ 4,080
Restricted share units ("RSUs")	2,209	995	3,653	1,925
Performance share units ("PSUs")	759	1,577	1,447	2,849
Total	**$ 5,714**	**$ 4,576**	**$ 10,054**	**$ 8,854**

As of June 30, 2020, the unrecognized share-based compensation expense related to unvested awards under the 2014 Plans were:

	Unrecognized share-based compensation expense	Weighted average remaining period for recognition
	(in thousands)	(in years)
Award type		
Share options	$ 27,450	3.00
Restricted share units	15,705	2.28
Performance share units	3,519	1.37
Total	**$ 46,674**	**2.63**

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

Share options

The following table summarizes option activity for the six months ended June 30, 2020:

	Options	
	Number of ordinary shares	Weighted average exercise price
Outstanding at December 31, 2019	**2,683,104**	**$ 21.29**
Granted	470,327	$ 53.26
Forfeited	(69,758)	$ 36.13
Exercised	(203,940)	$ 16.68
Outstanding at June 30, 2020	**2,879,733**	**$ 26.47**
Thereof, fully vested and exercisable at June 30, 2020	1,530,724	$ 16.06
Thereof, outstanding and expected to vest after June 30, 2020	1,349,009	$ 38.30

Total weighted average grant date fair value of options issued during the period (in $ millions)	$ 14.2
Proceeds from option sales during the period (in $ millions)	$ 3.4

Share options are priced on the date of grant and, except for certain grants made to non-executive directors, vest over a period of four years, the first 25% vests after one year from the grant date and the remainder vests in equal quarterly installments, over years two, three and four. Any options that vest must be exercised by the tenth anniversary of the grant date.

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

	Three months ended June 30,		Six months ended June 30,	
Assumptions	**2020**	**2019**	**2020**	**2019**
Expected volatility	70%	75%	70%	75%
Expected terms	10 years	10 years	10 years	10 years
Risk free interest rate	0.76% - 0.83%	2.17% - 2.63%	0.76% - 1.44%	2.17% - 2.87%
Expected dividend yield	0%	0%	0%	0%

Restricted share units (“RSUs”)

The following table summarizes the RSUs activity for the six months ended June 30, 2020:

	RSU	
	Number of ordinary shares	Weighted average grant-date fair value
Non-vested at December 31, 2019	**370,830**	**$ 28.62**
Granted	244,449	$ 52.81
Vested	(171,401)	$ 19.36
Forfeited	(14,562)	$ 45.12
Non-vested at June 30, 2020	**429,316**	**$ 45.53**

Total weighted average grant date fair value of RSUs granted during the period (in $ millions)	$ 12.9

RSUs vest over one to three years. RSUs granted to non-executive directors vest one year from the date of grant.

Performance share units ("PSUs")

The following table summarizes the PSUs activity for the six months ended June 30, 2020:

	PSU	
	Number of ordinary shares	Weighted average grant-date fair value
Non-vested at December 31, 2019	**479,422**	**$ 21.17**
Granted	91,003	$ 57.56
Vested	(354,105)	$ 17.44
Non-vested at June 30, 2020	**216,320**	**$ 42.58**
Total weighted average grant date fair value of PSUs granted during the period (in $ millions)		$ 5.2

In January 2019, the Company awarded PSUs to its executives and certain other members of senior management. These PSUs were earned as of January 2020 based on an assessment by the Company's board of directors of the level of achievement of agreed upon performance targets through December 31, 2019. PSUs vest after three years.

b) Employee Share Purchase Plan ("ESPP")

In June 2018, the Company's shareholders adopted and approved an ESPP allowing the Company to issue up to 150,000 ordinary shares. The ESPP is intended to qualify under Section 423 of the Internal Revenue Code of 1986. Under the ESPP, employees are eligible to purchase ordinary shares through payroll deductions, subject to any plan limitations. The purchase price of the ordinary shares on each purchase date is equal to 85% of the lower of the closing market price on the offering date and the closing market price on the purchase date of each three-month offering period. During the six months ended June 30, 2020, 3,005 ordinary shares were issued under the ESPP compared to 4,870 during the same period in 2019. As of June 30, 2020, a total of 135,202 ordinary shares remains available for issuance under the ESPP plan compared to a total of 142,539 as of June 30, 2019.

9 Income taxes

Deferred tax assets and deferred tax liabilities are recognized based on the expected future tax consequences of temporary differences between the financial statement carrying amounts and the income tax basis of assets and liabilities, using current statutory rates. A valuation allowance is recorded against deferred tax assets if it is more likely than not that some or all the deferred tax assets will not be realized. The Company reassessed the need for a full valuation allowance in conjunction with entering into the CSL Behring Agreement. Closing of the CSL Behring Agreement is contingent on the successful completion of reviews under the antitrust laws in the United States, Australia, and the United Kingdom, which has not occurred to date. Closing of the transaction is dependent on the timing, extent and result of the regulatory review process. In its assessment of whether or not it was more likely than not that the Company's deferred tax assets will be realized, the Company considered all relevant facts and circumstances, including in particular similar regulatory reviews as well as the three-year cumulative losses reported by the Company. The Company concluded that it should continue to record a full valuation allowance as of June 30, 2020. As of December 31, 2019, the Company's valuation allowance amounted to $109.9 million.

10 Basic and diluted earnings per share

Diluted earnings per share are calculated by adjusting the weighted average number of ordinary shares outstanding, assuming conversion of all potentially dilutive ordinary shares. As the Company has incurred a loss, all potentially dilutive ordinary shares would have an antidilutive effect, if converted, and thus have been excluded from the computation of loss per share. The shares are presented without giving effect to the application of the treasury method or exercise prices that would be above the share price as of June 30, 2020 and June 30, 2019, respectively. In addition, the BMS warrants were not exercisable as of these dates since this would have required the prior designation of Collaboration Targets by BMS. This would generally result in a lower number of potentially dilutive ordinary shares as some stock option grants as well as the BMS warrants would have been excluded.

The potentially dilutive ordinary shares are summarized below:

	June 30,	
	2020	2019
	(ordinary shares)	
BMS warrants	8,060,500	8,435,000
Stock options under 2014 Plans	2,879,733	2,943,756
Non-vested RSUs and earned PSUs	645,636	842,859
Stock options under previous option plan	14,000	14,000
Employee share purchase plan	254	596
Total potential dilutive ordinary shares	**11,600,123**	**12,236,211**

11 Subsequent events

None.

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 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, 2019, which was filed with the Securities and Exchange Commission (the "SEC"), on March 2, 2020. Our unaudited consolidated financial statements have been prepared in accordance with generally accepted accounting principles in the U.S. ("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, including product candidates for the treatment of hemophilia B, which we intend to license to CSL Behring pursuant to the CSL Behring Agreement (as defined below), and 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, current good manufacturing practices ("cGMP")-compliant, manufacturing process. We believe our Lexington, Massachusetts-based facility is one of the world's leading, most versatile, gene therapy manufacturing facilities.

Business Developments

Below is a summary of our recent significant business developments:

CSL Behring commercialization and license agreement

On June 24, 2020, uniQure biopharma B.V., our wholly-owned subsidiary of uniQure N.V., entered into a commercialization and license agreement (the "CSL Behring Agreement") with CSL Behring LLC ("CSL Behring") providing CSL Behring exclusive global rights to etranacogene dezaparvovec, our investigational gene therapy for patients with hemophilia B (the "Product").

Under the terms of the CSL Behring Agreement, we will receive a $450 million upfront cash payment upon the closing of the CSL Behring Agreement and be eligible to receive up to $1.6 billion in payments based on regulatory and commercial milestones. We will also be eligible to receive tiered double-digit royalties in a range of up to a low-twenties percent of net sales of the Product based on sales thresholds.

Pursuant to the CSL Behring Agreement, we will be responsible for the completion of the HOPE-B clinical trial, manufacturing process validation, and the manufacturing supply of Product until such time that these capabilities may be transferred to CSL Behring or its designated contract manufacturing organization. Pursuant to a development and commercial supply agreement, we will supply Product to CSL Behring at an agreed-upon price per the contract. We and CSL Behring executed the development and commercial supply agreement simultaneously with the CSL Behring Agreement. Certain provisions will not become effective until after we receive regulatory approval to close the transaction. Clinical development and regulatory activities performed by us pursuant to the CSL Behring Agreement will be reimbursed by CSL Behring. CSL Behring will be responsible for global regulatory submissions and commercialization requirements for Product.

Other than under the CSL Behring Agreement, neither we nor CSL Behring may perform any clinical trials, with the exception of trials required to extend the label or gain marketing authorization outside the United States or the European Union, for any gene therapy product, gene-editing product, or any other product comprising an AAV vector to conduct nucleotide transfer (including DNA and RNA) for the treatment, prevention, or cure of Hemophilia B for a period commencing on June 24, 2020 and continuing for a period of four years following the first commercial sale of the Product in the United States, and neither we nor CSL Behring may commercialize such a product for a period commencing as of June 24, 2020 and continuing for a period of seven years following the first commercial sale of the Product in the United States. This exclusivity commitment would not bind an acquirer of us that owns or controls such a product so long as certain precautions are followed to ensure that CSL Behring's confidential information and our proprietary technology related to Product are not used or accessed by personnel of such acquirer who are developing or commercializing such competing product.

Unless earlier terminated as described below, the CSL Behring Agreement will continue on a country-by-country basis until expiration of the royalty term in a country. The royalty term expires in a country on the later of (a) 15 years after the first commercial sale of Product in such country, (b) expiration of regulatory exclusivity for Product in such country and (c) expiration of all valid claims of specific licensed patents covering Product in such country. Either we or CSL Behring may terminate the CSL Behring Agreement for the other party's material breach, if such breach is not cured within a specified cure period. In addition, if CSL Behring fails to commercialize Product in any of a group of major countries for an extended period of time following the first regulatory approval of Product in any of such group of countries (other than due to certain specified reasons) and such failure has not been cured within a specified cure period, then we may terminate the CSL Behring Agreement. CSL Behring may also terminate the CSL Behring Agreement for convenience.

Closing of the CSL Behring Agreement is contingent on the successful completion of reviews under antitrust laws in the United States, Australia, and the United Kingdom, which has not occurred to date. Closing of the transaction is dependent on the timing, extent, and result of the regulatory review process. We do not believe that the consummation of the transaction will result in a violation of any applicable antitrust laws. However, there can be no assurance that a challenge on antitrust grounds will not be made, or if such a challenge is made, what the result would be. In accordance with our existing license and other agreements we are contractually required to pay in total a low to high single digit percentage of any upfront payment to our licensors and financial advisor ("License Fees").

As of June 30, 2020, we concluded we have no enforceable right to receive any of the upfront payment, the regulatory and sales milestone payments or the royalties (together "CSL License Revenue") that we may receive in accordance with the CSL Behring Agreement, as all payments are contingent upon the successful completion of reviews under antitrust laws in the United States, Australia and the United Kingdom. Therefore, we determined we will not recognize any revenue in relation to the CSL License Revenue, in accordance with ASC 606.

We determined that in accordance with Dutch taw law we will recognize the CSL Behring License Revenue as well as the License Fees as taxable results as of the date on which we are contractually entitled to receive (or obligated to make) a payment under the CSL Behring Agreement. We expect to continue to incur taxable losses in the Netherlands except for the period in which we receive the $450 million upfront payment. In the event that we recognize the $450 million upfront payment in 2020, such payment will be subject to Dutch corporate income tax at a rate of 25.0%. Any CSL License Revenue including the upfront payment that we recognize thereafter will be subject to Dutch corporate income tax at a rate of 21.7%. However, we do not expect that we will be required to pay any income taxes in the period in which we recognize the $450 million upfront payment as taxable revenue, as such payment is not expected to exceed the net operating losses that we have carried forward in the Netherlands. We have historically recorded a full valuation allowance against our net deferred tax assets.

Future taxable income or losses and a potential reversal of the valuation allowance will be impacted by a variety of factors, of which some are outside of our control. Such factors include the outcome and timing of the reviews under antitrust laws in the United States, Australia, and the United Kingdom, the change in the Dutch corporate income tax rate in 2021, additional net operating losses we may generate in relation to the CSL Behring Agreement, and the amount of License Fees we are required to pay.

We recognize deferred tax assets to the extent that we determine that these assets are more likely than not to be realized. In making such a determination, we weighed all available positive and negative evidence, including future income projections from the CSL Behring Agreement, and concluded that it is more likely than not that the deferred tax assets will not be realized. Accordingly, we continued to record a full valuation allowance as of June 30, 2020.

Hemophilia B program – Etranacogene dezaparvovec (AMT-061)

In June 2018, we initiated our Phase III HOPE-B pivotal trial of etranacogene dezaparvovec (the "HOPE-B trial"). The HOPE-B trial is a multinational, multi-center, open-label, single-arm study to evaluate the safety and efficacy of etranacogene dezaparvovec. After a six-month lead-in period, patients received a single intravenous administration of etranacogene dezaparvovec. The primary endpoint of the study is based on the FIX activity level achieved following the administration of etranacogene dezaparvovec, and the secondary endpoints are measuring annualized FIX replacement therapy usage, annualized bleed rates and safety. Patients enrolled in the HOPE-B trial were tested for the presence of pre-existing neutralizing antibodies to AAV5 but not excluded from the trial based on their titers.

In March 2020, we completed dosing of 54 patients in the HOPE-B trial. The targeted number of patients to be dosed per the clinical trial protocol was 50. As set forth below, we have implemented and continue to implement various measures to allow us to closely monitor the trial within the guidance provided by the FDA regarding the impact of the COVID-19 coronavirus pandemic ("COVID-19") in order to minimize any risk or disruption in patient follow-up visits.

In August 2018, we initiated a Phase IIb dose-confirmatory study of etranacogene dezaparvovec. In December 2019, we presented 52-week follow-up data from the study showing that all three patients had stabilized and sustained FIX activity at therapeutic levels after a one-time administration of etranacogene dezaparvovec. Mean FIX activity for the three patients at 52 weeks after administration was 41% of normal, with the first patient achieving FIX activity of 50% of normal, the second patient achieving FIX activity of 31% of normal and the third patient achieving FIX activity of 41% of normal. The second and third patients had previously screen-failed and were excluded from another gene therapy study due to pre-existing neutralizing antibodies to a different AAV vector.

Etranacogene dezaparvovec has been granted Breakthrough Therapy Designation by the United States Food and Drug Administration ("FDA") and access to the PRIME initiative by the European Medicines Agency ("EMA").

Huntington's disease program (AMT-130)

AMT-130 is our novel gene therapy candidate for the treatment of Huntington's disease. AMT-130 utilizes our miQURE proprietary, gene-silencing platform and incorporates an AAV vector carrying a miRNA specifically designed to silence the huntingtin gene and the potentially highly toxic exon 1 protein fragment. AMT-130 has received orphan drug and fast track designations from the FDA and Orphan Medicinal Product Designation from the EMA.

In June 2020, we announced the completion of the first two patient procedures in the Phase I/II clinical trial of AMT-130 for the treatment of Huntington's disease. These procedures occurred after a postponement that resulted from the COVID-19 pandemic and the associated states of emergency declarations in the United States. The Phase I/II protocol is a randomized, imitation surgery-controlled, double-blinded study conducted at three surgical sites, and multiple referring, non-surgical sites in the U.S. The primary objective of the study is to evaluate the safety, tolerability, and efficacy of AMT-130 at two doses.

AMT-150 for Spinocerebellar Ataxia type 3 (SCA3)

In May 2020, we presented preclinical data at the American Society of Gene and Cell Therapy ("ASGCT") Annual Meeting, on our gene therapy candidate SCA3. In an in vivo preclinical study, six non-human primates (NHP) received a one-time injection of AMT-150 via the cisterna magna to assess expression and distribution. Samples taken after eight weeks showed widespread transduction of the brain and spinal cord, with the highest genome copies found in the posterior fossa and cortical regions. In other preclinical studies, researchers evaluated AMT-150 in SCA3 mouse models, as well as human induced pluripotent stem cell ("iPSC")-derived neurons and astrocytes, to investigate potential off-target effects of AAV5-miATXN3. The iPSC-derived cell cultures, which were derived from two SCA3 patients, represent the most disease-relevant cell type for therapeutic targeting of AMT-150. A clear dose-dependent expression of miATXN3 was observed in the iPSC-derived neurons and astrocytes transduced with AMT-150. Mature miATXN3 molecules were also associated with extracellular vesicles that strongly correlated with the dose and miATXN3 expression, suggesting the potential therapeutic spread of the engineered miATXN3. Additionally, AMT-150 demonstrated ATXN3 knockdown in human neurons and various SCA3 mouse models with subsequent neuropathology improvement.

AMT-190 for Fabry disease

In May 2020, we presented preclinical data at the ASGCT Annual Meeting on our gene therapy candidate AMT-190 for Fabry disease. In vivo studies in wild-type NHPs were conducted to assess expression of a proprietary, exclusively licensed modified NAGA ("ModNAGA") upon AAV-injection. These studies demonstrated that a single administration of AMT-190 resulted in ModNAGA expression in the liver and significant increases of GLA activity levels in the NHP plasma.

AMT-180 for Hemophilia A

In June 2020, we announced that we plan to de-prioritize our research program of AMT-180 for patients with hemophilia A, as part of our effort to focus on those gene therapy programs that have the greatest potential to improve patients' lives and generate long-term value for shareholders.

COVID-19 measures

In December 2019, COVID-19, a novel coronavirus disease, was reported and in January 2020, the World Health Organization ("WHO") declared it a Public Health Emergency of International Concern. On February 28, 2020, the WHO raised its assessment of the COVID-19 threat from high to very high at a global level due to the continued increase in the number of cases and affected countries, and on March 11, 2020, the WHO characterized COVID-19 as a pandemic.

Starting March 2020, we implemented measures to address the impact of COVID-19 on our business. As of March 13, 2020, we mandated a work-from-home policy for all non-essential employees at our Amsterdam and Lexington facilities. We supported our employees in setting up a healthy and efficient remote working environment. In conjunction with implementing this policy, we accelerated the roll-out of a number of information technology security measures such as dual factor authentication, to address the increased risks that we might be exposed to in conjunction with our employees working remotely. In addition, we conducted awareness training around cybersecurity for critical functions involved in making payments to vendors such as finance and supply chain.

As a biopharma research and development company, we are deemed to provide essential services under the "stay at home" advisory that was issued by the Governor of Massachusetts on March 23, 2020 and we are therefore maintaining our manufacturing operations at our Lexington site. We adapted to operate our laboratories at our Amsterdam site to comply with social distancing rules and to ensure the health and wellbeing of our employees under the current circumstances. We had discouraged our employees from using public transport and are accommodating them by arranging for alternative means of transport until June 1, 2020 in line with policies advised by the relevant Dutch authorities.

We conduct frequent status video-meetings of local management at our two sites as well as leadership-team video meetings to implement these measures and to monitor the evolving situation. In addition, we inform our employees through periodic newsletters and have organized virtual local and global townhalls to share information and provide direction and support to our employees.

We have adapted our ongoing clinical research activities based on the directions and flexibility provided by the "FDA Guidance on Conduct of Clinical Trials of Medical Products during COVID-19 Pandemic" issued on March 18, 2020 in an attempt to minimize any risk, disruption or delay in either patient dosing or follow-up visits.

We are starting to reopen the Amsterdam and Lexington facilities in phases, in line with the reopening plans that are prescribed by the local government. Starting June 1, 2020, we encouraged our Amsterdam employees to work a minimum of two days per week from the office and approximately 50% of local staff has been working on site since then. Our employees that cannot perform their duties outside of our Lexington facility continue to work at our Lexington facility. Other employees have returned to our Lexington facility on a limited basis, and all employees are encouraged to work from home to the extent possible. To ensure adequate social distancing in our Lexington facility, our COVID-19 protocols currently limit occupancy to numbers below those allowed by the Massachusetts COVID-19 guidelines. Our occupancy at the Lexington facility has been less than approximately 20% of our permitted occupancy during all phases of the Massachusetts reopening plan.

The broader implications of COVID-19 on our results of operations and overall financial performance remain uncertain. The COVID-19 pandemic and its adverse effects have become more prevalent in the locations where we, and our third-party business partners conduct business. While we have experienced disruptions in our operations as a result of COVID-19, we are adapting to the current environment to minimize the effect to our business. However, we may experience more pronounced disruptions in our operations in the future.

We believe our cash and cash equivalents as of June 30, 2020, will enable us to fund our operating expenses including our debt repayment obligations as they become due and capital expenditure requirements into 2022. Upon the receipt of the $450 million payment due on the closing of the CSL Behring Agreement, we expect that our cash and cash equivalents will be sufficient to fund operations into the second half of 2024. The closing of the transactions contemplated by the CSL Behring agreement is contingent on the successful completion of reviews under antitrust laws in the United States, Australia, and the United Kingdom, which has not occurred to date. The transactions contemplated by the CSL Behring Agreement are expected to materially impact our profitability and cash flows. We expect to generate positive cash flows in the period of closing and to recognize material revenue related to the CSL Behring Agreement. However, we expect to continue to incur losses and to generate negative cash flows outside the fiscal year in which we close the transactions contemplated by the CSL Behring Agreement. We have no firm sources of additional funding. Until such time, if ever, as we can generate substantial cash flows from successfully commercializing our proprietary 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. Similar to the other risk factors pertinent to our business, COVID-19 might unfavorably impact our ability to generate such additional funding.

BMS collaboration

We entered into a collaboration and license agreement with Bristol-Myers Squibb (“BMS”) in May 2015. We have been supporting BMS in the discovery, non-clinical, analytical and process development efforts of our gene therapy technology platform for the research, development and commercialization of therapeutics aimed at multiple targets in cardiovascular and other diseases (“Collaboration Targets”). For any Collaboration Targets that are advanced, we will be responsible for manufacturing of clinical and commercial supplies using our vector technologies and industrial, proprietary insect-cell based manufacturing platform. BMS has reimbursed us for all our research and development costs in support of the collaboration during the initial research term. BMS will lead the development, regulatory and commercial activities for all four currently active Collaboration Targets as well as additional Collaboration Targets that may be advanced.

In February 2019, BMS requested a one-year extension of the research term. In April 2019, following an assessment of the progress of this collaboration and our expanding proprietary programs, we notified BMS that we did not intend to agree to an extension of the research term. Accordingly, the initial four-year research term under the collaboration terminated on May 21, 2019. The Company has agreed to certain restrictions on its ability to work independently of the collaboration, either directly or indirectly through any affiliate or third party, on certain programs that would be competitive with the collaboration programs. We are currently in discussions with BMS potentially to amend the collaboration and license agreement and other related agreements following the expiration of the research term. The final resolution of these discussions may or may not result in material changes to our collaboration with BMS.

Organization

On June 17, 2020, our shareholders voted to approve the appointment of Leonard E. Post, Ph.D. as a non-executive director of the Board of Directors. Dr. Post replaced Dr. David Schaffer, whose term as a non-executive director of the Board of Directors ended on the same date.

Intellectual Property

On January 4, 2020, a petition seeking *Inter Partes Review* of U.S. Patent No. 9,249,405 (the “’405 Patent”) was filed by Pfizer, Inc. The petition sought to invalidate claims 6 and 9-15 of the ‘405 Patent. On April 17, 2020, we filed our preliminary response to the petition, disclaiming claims 6 and 9-13 of the ‘405 patent and otherwise requesting the denial of the petition. On July 13, 2020, the United States Patent and Trademark Office issued its decision to institute the *Inter Partes Review*. We are in the process of preparing our response to the petition, which is due October 5, 2020 under the schedule set for the proceeding.

Financial Overview

Key components of our results of operations include the following:

	Three months ended June 30,		Six months ended June 30,	
	2020	2019	2020	2019
	(in thousands)		(in thousands)	
Total revenues	$ 1,535	$ 2,474	$ 1,639	$ 3,610
Research and development expenses	(28,401)	(24,154)	(54,414)	(44,691)
Selling, general and administrative expenses	(11,511)	(7,870)	(20,583)	(15,937)
Net loss	(42,551)	(31,399)	(70,550)	(59,171)

As of June 30, 2020, and December 31, 2019, we had cash and cash equivalents of $314.3 million and $377.8 million, respectively. We had a net loss of $42.6 million and $70.6 million in the three and six months ended June 30, 2020, compared to $31.4 million and $59.2 million for the same period in 2019. As of June 30, 2020, and December 31, 2019, we had accumulated deficits of $730.3 million and $659.7 million, respectively. Our losses will be materially impacted by the amount of license revenue that we will recognize in accordance with ASC 606 following the potential closing of our collaboration and license agreement with CSL Behring.

We anticipate that our expenses will increase substantially as we:

- Advance the clinical development of AMT-130 for our Huntington's disease gene therapy program;
- Build-out our commercial and medical affairs infrastructure and seek marketing approval for any product candidates (including etranacogene dezaparvovec in case our collaboration and license agreement with CSL Behring would not close) that successfully complete clinical trials;
- Advance multiple research programs related to gene therapy candidates targeting liver-directed and central nervous system ("CNS") diseases;
- Continue to expand, enhance and optimize our technology platform, including our manufacturing capabilities, next-generation viral vectors and promoters, and other enabling technologies;
- Continue to expand our employee base to support research and development, as well as general and administrative functions;
- Acquire or in-license rights to new therapeutic targets or product candidates; and
- Maintain, expand and protect our intellectual property portfolio, including in-licensing additional intellectual property rights from third parties.

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

Critical Accounting Policies and Estimates

In preparing our consolidated financial statements in accordance with U.S. GAAP and pursuant to the rules and regulations promulgated by the SEC we make 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 implementation of ASC 842 Leases, recognition of License Revenue in accordance with ASC 606, BMS warrants, share-based payments and corporate income taxes related to valuation allowance. We base our assumptions, judgments and estimates on historical experience and various other factors that we believe to be reasonable under the circumstances, the results of which form the basis for making judgments about the carrying values of assets and liabilities that are not clear from other sources. Actual results may differ from these estimates under different assumptions or conditions. In making estimates and judgments, management employs critical accounting policies. During the six months ended June 30, 2020, 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, 2019, which was filed with the SEC on March 2, 2020.

We believe that the assumptions, judgments and estimates involved in the recognition of License Revenue in accordance with ASC 606, BMS warrants, share-based payments, corporate income taxes related to valuation allowance and accounting for operating leases under ASC 842 to be our critical accounting policies.

The preparation of our consolidated financial statements for the three- and six-month period ended June 30, 2020, required us to analyze the accounting treatment of the CSL Behring Agreement. This analysis required us to exercise judgement regarding the timing and the likelihood of closing the transaction.

Closing of the CSL Behring Agreement is contingent on the successful completion of reviews under antitrust laws in the United States, Australia, and the United Kingdom, which has not occurred to date. Closing of the transaction is dependent on the timing, extent and result of the regulatory review process. We do not believe that the consummation of the transaction will result in a violation of any applicable antitrust laws. However, there can be no assurance that a challenge on antitrust grounds will not be made, or if such a challenge is made, what the result would be.

As of June 30, 2020, we concluded we have no enforceable right to receive any of CSL License Revenue that we may receive in accordance with the CSL Behring Agreement, as all payments are contingent upon the successful completion of reviews under antitrust laws in the United States, Australia and the United Kingdom. Therefore, we determined we will not recognize any revenue in relation to the CSL License Revenue, in accordance with ASC 606.

We recognize deferred tax assets to the extent that we determine that these assets are more likely than not to be realized. In making such a determination, we weighed all available positive and negative evidence, including future income projections from the CSL Behring Agreement, and concluded that it is more likely than not that the deferred tax assets will not be realized. Accordingly, we continued to record a full valuation allowance as of June 30, 2020.

Revenues

We recognize Collaboration Revenues associated with pre-clinical Collaboration Target specific, non-clinical, analytical and process development activities that are reimbursable by BMS under our collaboration and license agreement during the initial research term, which ended on May 21, 2019. We are currently in discussions with BMS potentially to amend the BMS CLA and other related agreements following the expiration of the research term. During these discussions, which may be terminated by us or BMS at any time, we have agreed, subject to certain conditions, to continue providing limited support of the pre-clinical Collaboration Targets, and any related costs will be reimbursed by BMS.

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, when earned.

Research and development expenses

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

- Employee-related expenses, including salaries, benefits, travel and share-based compensation expense;
- Costs incurred for laboratory research, preclinical and nonclinical studies, clinical trials, statistical analysis and report writing, and regulatory compliance costs incurred with clinical research organizations and other third-party vendors;
- Costs incurred to conduct consistency and comparability studies;
- Costs incurred for the development and improvement of our manufacturing processes and methods;
- Costs associated with our research activities for our next-generation vector and promoter platform; and
- Facilities, depreciation and other expenses, which include direct and allocated expenses for rent and maintenance of facilities, insurance and other supplies.

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, including as a result of the COVID-19 pandemic, 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, consulting, 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. Our selling costs include employee expenses as well as professional fees related to the preparation of a commercial launch of etranacogene dezaparvovec.

Other items, net

Our other income consists of payments to subsidize our research and development efforts as well as income from the subleasing of our Amsterdam facility.

Our other expense consists of expenses we incur in relation to our subleasing income.

Results of Operations

Comparison of the three months ended June 30, 2020 and 2019

The following table presents a comparison of the three months ended June 30, 2020 and 2019.

	Three months ended June 30,		
	2020	2019	2020 vs 2019
		(in thousands)	
Total revenues	$ 1,535	$ 2,474	$ (939)
Operating expenses:			
Research and development expenses	(28,401)	(24,154)	(4,247)
Selling, general and administrative expenses	(11,511)	(7,870)	(3,641)
Total operating expenses	**(39,912)**	**(32,024)**	**(7,888)**
Other income	669	566	103
Other expense	(500)	(347)	(153)
Loss from operations	**(38,208)**	**(29,331)**	**(8,877)**
Other non-operating items, net	(4,343)	(2,068)	(2,275)
Net loss	**$ (42,551)**	**$ (31,399)**	**$ (11,152)**

Revenue

Our revenue for the three months ended June 30, 2020 and 2019 was as follows:

	Three months ended June 30,		
	2020	2019	2020 vs 2019
		(in thousands)	
License Revenue	$ 1,530	$ 2,108	$ (578)
Collaboration Revenue	5	366	(361)
Total revenues	**$ 1,535**	**$ 2,474**	**$ (939)**

We recognize License Revenue related to upfront payments and target designation fees received from BMS in 2015. We recognized $1.5 million License Revenue in the three months ended June 30, 2020, compared to $2.1 million for the same period in 2019. The reduction in License Revenue is primarily a result of delays in development timelines due to our ongoing discussions surrounding the potential amendment of the BMS CLA. Continuing these discussions to some extent was impacted by the parties' focus to adjust their operations to COVID-19.

We recognized $0.0 million Collaboration Revenue in the three months ended June 30, 2020, compared to $0.4 million for the same period in 2019. The decrease in Collaboration Revenue was primarily related to the reduction of activities following the termination of the initial Research Term under the BMS CLA in May 2019.

Research and development expenses

Research and development expenses for the three months ended June 30, 2020 were $28.4 million, compared to $24.2 million for the same period in 2019. Other research and development expenses are separately classified in the table below. These are not allocated as they are deployed across multiple projects under development.

	Three months ended June 30,		
	2020	2019	2020 vs 2019
		(in thousands)	
Etranacogene dezaparvovec (AMT-060/061)	$ 4,673	$ 4,363	$ 310
Huntington's disease (AMT-130)	2,095	1,164	931
Programs in preclinical development and platform related expenses	1,770	1,546	224
Total direct research and development expenses	**$ 8,538**	**$ 7,073**	**$ 1,465**
Employee and contractor-related expenses	9,990	8,239	1,751
Share-based compensation expense	2,894	2,111	783
Facility expenses	4,185	3,625	560
Disposables	2,501	2,662	(161)
Other expenses	293	444	(151)
Total other research and development expenses	**$ 19,863**	**$ 17,081**	**$ 2,782**
Total research and development expenses	**$ 28,401**	**$ 24,154**	**$ 4,247**

Direct research and development expenses

Etranacogene dezaparvovec (AMT-060/061)

In the three months ended June 30, 2020 and 2019, the external costs for our hemophilia B program were primarily related to the execution of our Phase III clinical trial. We enrolled patients into a six-month lead in phase between January 2018 and September 2019 and dosed a total of 54 patients between January 2019 and March 2020. Our expenses related to etranacogene dezaparvovec were largely unaffected by the COVID-19 pandemic as we completed enrollment immediately prior to the lockdowns in those countries in which we enroll patients. We have implemented additional measures to minimize any risk, disruption or delay on follow-up visits.

In addition, we continue to incur costs for the long-term follow-up of patients in our Phase I/II clinical trial of AMT-060 and our Phase IIb clinical trial of etranacogene dezaparvovec.

Huntington disease (AMT-130)

In the three months ended June 30, 2020 and 2019, our external costs for the development of Huntington’s disease were primarily related to the execution of our Phase I/II clinical trial as well as expenses related to the procedures of the first two patients in June 2020. The procedure-related expenses were delayed as a result of the temporary postponement of the procedures due to the COVID-19 related state of emergency declarations in the United States.

Preclinical programs & platform development

In the three months ended June 30, 2020, we incurred $1.8 million of costs primarily related to our preclinical activities primarily associated with product candidates for Hemophilia A (AMT-180), SCA3 (AMT-150) and Fabry disease (AMT-190), as well as various other research programs and technology innovation projects, compared to $1.5 million in the same period in 2019.

Other research & development expenses

- We incurred $10.0 million in personnel and contractor related expenses in the three months ended June 30, 2020, compared to $8.2 million for the same period in 2019. Our costs during the three months ended June 30, 2020 increased by $1.8 million as a result of the recruitment of personnel to support the development of our product candidates;
- We incurred $2.9 million in share-based compensation expenses in the three months ended June 30, 2020, compared to $2.1 million for the same period in 2019; and
- We incurred $4.2 million in operating expenses and depreciation expenses related to our rented facilities in the three months ended June 30, 2020, compared to $3.6 million in the same period in 2019. Our costs during the three months ended June 30, 2020, primarily increased as a result of extending and expanding (as from June 2019) the lease of our Lexington facility.

Selling, general and administrative expenses

Selling, general and administrative expenses for the three months ended June 30, 2020 were $11.5 million, compared to $7.9 million for the same period in 2019.

- We incurred $2.9 million in personnel and contractor related expenses in the three months ended June 30, 2020, compared to $2.4 million in the same period in 2019;
- We incurred $2.8 million in share-based compensation expenses in the three months ended June 30, 2020, compared to $2.4 million in the same period in 2019; and
- We incurred $3.0 million in professional fees in the three months ended June 30, 2020, compared to $1.3 million in the same period in 2019. The increase was primarily driven by corporate initiatives.

Our selling, general and administrative expenses were largely unaffected by the COVID-19 pandemic.

Other items, net

We recognized $0.3 million in income from payments received from European authorities to subsidize our research and development efforts in the Netherlands in the three months ended June 30, 2020, compared to $0.3 million for the same period in 2019.

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

Our other non-operating items, net, for the three months ended June 30, 2020 and 2019 were as follows:

	Three months ended June 30,		
	2020	2019	2020 vs 2019
		(in thousands)	
Interest income	$ 81	$ 728	$ (647)
Interest expense - Hercules long-term debt	(970)	(937)	(33)
Foreign currency losses, net	(3,645)	(1,252)	(2,393)
Other non-operating gains / (losses)	191	(607)	798
Total other non-operating loss, net	**$ (4,343)**	**$ (2,068)**	**$ (2,275)**

We recognized a net foreign currency loss related to our borrowings from Hercules and our cash and cash equivalents of $3.6 million during the three months ended June 30, 2020, compared to a net loss of $1.3 million during the same period in 2019.

In the three months ended June 30, 2020, we recognized income of $0.2 million related to changes in the fair value of the BMS warrants compared to a loss of $0.6 million related to changes in the fair value of the BMS warrants for the same period in 2019.

Comparison of the six months ended June 30, 2020 and 2019

The following table presents a comparison of the six months ended June 30, 2020 and 2019.

	Six months ended June 30,		
	2020	2019	2020 vs 2019
	(in thousands)		
Total revenues	$ 1,639	$ 3,610	$ (1,971)
Operating expenses:			
Research and development expenses	(54,414)	(44,691)	(9,723)
Selling, general and administrative expenses	(20,583)	(15,937)	(4,646)
Total operating expenses	**(74,997)**	**(60,628)**	**(14,369)**
Other income	1,526	879	647
Other expense	(839)	(696)	(143)
Loss from operations	**(72,671)**	**(56,835)**	**(15,836)**
Non-operating items, net	2,121	(2,336)	4,457
Net loss	$ **(70,550)**	$ **(59,171)**	$ **(11,379)**

Revenue

Our revenue for the six months ended June 30, 2020 and 2019 was as follows:

	Six months ended June 30,		
	2020	2019	2020 vs 2019
	in thousands		
License revenue	$ 1,577	$ 2,665	$ (1,088)
Collaboration revenue	62	945	(883)
Total revenues	**$ 1,639**	**$ 3,610**	**$ (1,971)**

We recognize License Revenue related to upfront payments and target designation fees received from BMS in 2015. We recognized $1.6 million License Revenue in the six months ended June 30, 2020, compared to $2.7 million for the same period in 2019. The reduction in License Revenue is primarily a result of delays in development timelines due to our ongoing discussions surrounding the potential amendment of the BMS CLA as well as COVID-19. Continuing these discussions to some extent was impacted by the parties' focus to adjust their operations to COVID-19.

We recognized $0.1 million Collaboration Revenue in the six months ended June 30, 2020, compared to $0.9 million for the same period in 2019. The decrease in Collaboration Revenue was primarily related to the reduction of activities following the termination of the initial Research Term under the BMS CLA in May 2019.

Research and development expenses

Research and development expenses for the six months ended June 30, 2020 were $54.4 million, compared to $44.7 million for the same period in 2019. Other research and development expenses are separately classified in the table below. These are not allocated as they are deployed across multiple projects under development.

	Six months ended June 30,		
	2020	2019	2020 vs 2019
		(in thousands)	
Etranacogene dezaparvovec (AMT-060/061)	$ 9,213	$ 7,670	$ 1,543
Huntington's disease (AMT-130)	3,155	1,627	1,528
Programs in preclinical development and platform related expenses	3,225	2,435	790
Total direct research and development expenses	**$ 15,593**	**$ 11,732**	**$ 3,861**
Employee and contractor-related expenses	19,338	16,487	2,851
Share-based compensation expense	5,289	4,099	1,190
Facility expenses	8,201	6,919	1,282
Disposables	4,911	4,570	341
Other expenses	1,082	884	198
Total other research and development expenses	**$ 38,821**	**$ 32,959**	**$ 5,862**
Total research and development expenses	**$ 54,414**	**$ 44,691**	**$ 9,723**

Direct research and development expenses

Hemophilia B (AMT-060/061)

In the six months ended June 30, 2020 and 2019, the external costs for our hemophilia B program were primarily related to the execution of our Phase III clinical trial. We enrolled patients into a six-month lead in phase between January 2018 and September 2019 and dosed a total of 54 patients between January 2019 and March 2020. Our expenses related to etranacogene dezaparvovec were largely unaffected by the COVID-19 pandemic as we completed enrollment immediately prior to the lockdowns in those countries that we enroll patients. We have implemented additional measures to minimize any risk, disruption or delay on follow-up visits.

In addition, we continue to incur costs for the long-term follow-up of patients in our Phase I/II clinical trial of AMT-060 and our Phase IIb clinical trial of etranacogene dezaparvovec.

Huntington disease (AMT-130)

In the six months ended June 30, 2020 and 2019, our external costs for the development of Huntington’s disease were primarily related to the execution of our Phase I/II clinical trial as well as expenses related to the procedures of the first two patients in June 2020. The procedure-related expenses were delayed as a result of the temporary postponement of the procedures due to the COVID-19 related state of emergency declarations in the United States.

Preclinical programs & platform development

In the six months ended June 30, 2020, we incurred $3.2 million of costs primarily related to our preclinical activities associated with product candidates for Hemophilia A (AMT-180), SCA3 (AMT-150) and Fabry disease (AMT-190), as well as various other research programs and technology innovation projects, compared to $2.4 million in the same period in 2019.

Other research & development expenses

- We incurred $19.3 million in personnel and contractor related expenses in the six months ended June 30, 2020, compared to $16.5 million for the same period in 2019. Our costs during the six months ended June 30, 2020 increased by $2.8 million as a result of the recruitment of personnel to support the development of our product candidates;
- We incurred $5.3 million in share-based compensation expenses in the six months ended June 30, 2020, compared to $4.1 million for the same period in 2019; and
- We incurred $8.2 million in operating expenses and depreciation expenses related to our rented facilities in the six months ended June 30, 2020, compared to $6.9 million in the same period in 2019. Our costs during the six months ended June 30, 2020 primarily increased as a result of extending and expanding (as from June 2019) the lease of our Lexington facility.

Selling, general and administrative expenses

Selling, general and administrative expenses for the six months ended June 30, 2020 were $20.6 million, compared to $15.9 million for the same period in 2019.

- We incurred $6.1 million in personnel and contractor related expenses in the six months ended June 30, 2020, compared to $5.1 million in the same period in 2019;
- We incurred $4.8 million in share-based compensation expenses in the six months ended June 30, 2020, compared to $4.8 million in the same period in 2019; and
- We incurred $4.2 million in professional fees in the six months ended June 30, 2020, compared to $2.9 million in the same period in 2019. The decrease was primarily driven by timing of various corporate initiatives.

Our selling, general and administrative expenses were largely unaffected by the COVID-19 pandemic.

Other items, net

We recognized $0.5 million in income from payments received from European authorities to subsidize our research and development efforts in the Netherlands in the six months ended June 30, 2020, compared to $0.3 million for the same period in 2019.

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 (expense) / income. Following the exercise of the warrants by Hercules in February 2019 we no longer recognize changes in the fair value of these warrants within other non-operating (expense) / income.

Our other non-operating items, net, for the six months ended June 30, 2020 and 2019 were as follows:

	Six months ended June 30,		
	2020	2019	2020 vs 2019
	(in thousands)		
Interest income	$ 903	$ 1,170	$ (267)
Interest expense	(1,945)	(1,894)	(51)
Foreign currency gains, net	957	1,022	(65)
Other non-operating gains / (losses), net	2,206	(2,634)	4,840
Total non-operating income / (loss), net	**$ 2,121**	**$ (2,336)**	**$ 4,457**

We recognized a net foreign currency gain related to our borrowings from Hercules and our cash and cash equivalents of $1.0 million during the six months ended June 30, 2020, compared to a net gain of $1.0 million during the same period in 2019.

In the six months ended June 30, 2020, we recognized income of $2.2 million related to changes in the fair value of the Hercules and BMS warrants compared to a loss of $2.6 million for the same period in 2019. Changes in the fair value of the Hercules and BMS warrants are primarily impacted by changes in our share price, whereby a decrease in share price generally results in a decrease of the fair value.

Financial Position, Liquidity and Capital Resources

As of June 30, 2020, we had cash, cash equivalents and restricted cash of $316.9 million. We currently expect that our cash and cash equivalents will be sufficient to fund operations into 2022. Upon the receipt of the $450 million payment due on the closing of the CSL Behring Agreement, we expect that our cash and cash equivalents will be sufficient to fund operations into the second half of 2024. The table below summarizes our consolidated cash flow data for the six months ended June 30, 2020, and 2019.

	Six months ended June 30,	
	2020	2019
	(in thousands)	
Cash, cash equivalents and restricted cash at the beginning of the period	$ 380,726	$ 237,342
Net cash used in operating activities	(62,944)	(50,657)
Net cash used in investing activities	(4,606)	(2,428)
Net cash generated from financing activities	3,549	3,221
Foreign exchange impact	223	(443)
Cash, cash equivalents and restricted cash at the end of period	**$ 316,948**	$ **187,035**

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 $42.6 million and $70.6 million during the three and six months ended June 30, 2020, compared to a net loss of $31.4 million and $59.2 million during the same periods in 2019. As of June 30, 2020, we had an accumulated deficit of $730.3 million.

Sources of liquidity

From our first institutional venture capital financing in 2006 through June 30, 2020, we funded our operations primarily through private and public placements of equity securities and convertible and other debt securities as well as payments from our collaboration partners.

On September 10, 2019, we completed a follow-on public offering of 4,891,305 ordinary shares at a public offering price of $46.00 per ordinary share, and on September 13, 2019, we completed the sale of an additional 733,695 ordinary shares at a public offering price of $46.00 per ordinary share pursuant to the exercise by the underwriters of the option to purchase additional ordinary shares, resulting in total gross proceeds to us of $258.8 million. The net proceeds from this offering were $242.7 million, after deducting underwriting discounts and commissions and other offering expenses payable by us. We deducted $0.6 million of expenses incurred related to this offering from additional paid-in capital in the accompanying consolidated balance sheets and reflected this within the proceeds from public offering of shares, net of issuance costs within the cash flows from financing activities.

On December 6, 2018, we signed an amendment to the Second Amended and Restated Loan and Security Agreement (the “2018 Amended Facility”) with Hercules that both refinanced our then-existing $20 million credit facility and provided us with an additional unconditional commitment of $15 million as well as a conditional commitment of $15 million that expired on June 30, 2020. At signing, we drew down an additional $15 million, for a total outstanding amount of $35 million. We have the right to draw another $15 million through June 30, 2020 subject to the terms of the 2018 Amended Facility.

The 2018 Amended Facility extends the loan's maturity date until June 1, 2023. The interest-only period was initially extended from November 2018 to January 1, 2021. The interest-only period was further extended to January 1, 2022 as a result of raising more than $90.0 million in equity financing in September 2019. As of June 30, 2020, $35 million was outstanding under the 2018 Amended Facility (December 31, 2019: $35 million). We are required to repay the facility in equal monthly installments of principal and interest between the end of the interest-only period and the maturity date. The variable interest rate is equal to the greater of (i) 8.85% or (ii) 8.85% plus the prime rate less 5.50%. Under the 2018 Amended Facility, we paid a facility fee equal to 0.50% of the $35,000,000 loan outstanding and will owe a back-end fee of 4.95% of the outstanding debt.

The $450 million upfront payment we expect to receive on the closing of the CSL Behring Agreement is expected to fund operations into the second half of 2024. Closing of the transaction is expected to materially impact our profitability and cash flows. We expect to generate positive cash flows in the period of closing and to recognize material revenue related to the CSL Behring Agreement. However, we expect to continue to incur losses and to generate negative cash flows outside the fiscal year in which we close the transaction. We have no firm sources of additional funding. Until such time, if ever, as we can generate substantial cash flows from successfully commercializing our proprietary 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 2018 Amended Facility 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 2018 Amended Facility 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 $62.9 million for the six months ended June 30, 2020, and consisted of a net loss of $70.6 million adjusted for non-cash items, including depreciation and amortization expense of $3.5 million, share-based compensation expense of $10.1 million, fair value gains on derivative financial instruments of $2.2 million, unrealized foreign exchange gain of $0.9 million, and an decrease in unamortized deferred revenue of $1.6 million. Net cash used in operating activities also included unfavorable changes in operating assets and liabilities of $1.3 million. These changes primarily related to a net decrease in accounts receivable and accrued income, prepaid expenses and other current assets of $1.1 million and a net decrease in accounts payable, accrued expenses and other liabilities of $2.3 million.

Net cash used in operating activities was $50.7 million for the six months ended June 30, 2019, and consisted of a net loss of $59.2 million adjusted for non-cash items, including depreciation and amortization expense of $3.2 million, share-based compensation expense of $8.9 million, fair value losses on derivative financial instruments of $2.6 million, unrealized foreign exchange gain of $0.4 million, and a decrease in unamortized deferred revenue of $2.7 million. Net cash used in operating activities also included unfavorable changes in operating assets and liabilities of $3.1 million. These changes primarily related to a net increase in accounts receivable and accrued income, prepaid expenses and other current assets of $2.8 million primarily driven by an increase in prepaid expenses related to our etranacogene dezaparvovec and AMT-130 trials.

Net cash used in investing activities

In the six months ended June 30, 2020, we used $4.6 million in our investing activities compared to $2.4 million for the same period in 2019.

	Six months ended June 30,	
	2020	2019
	(in thousands)	
Build out of Lexington site	$ (576)	$ (930)
Build out of Amsterdam site	(1,816)	(502)
Acquisition of licenses, patents and other rights	(2,214)	(996)
Total investments	**$ (4,606)**	**$ (2,428)**

Net cash generated from financing activities

During the six months ended June 30, 2020, we received $3.5 million from the exercise of options to purchase ordinary shares in relation to our share incentive plans compared to $2.7 million in the same period 2019.

Funding requirements

We believe our cash and cash equivalents as of June 30, 2020 will enable us to fund our operating expenses including our debt repayment obligations as they become due and capital expenditure requirements into 2022. The $450 million upfront payment we expect to receive on the closing of the CSL Behring Agreement would provide us with additional funding into the second half of 2024. Our future capital requirements will depend on many factors, including but not limited to:

- the closing of our collaboration and license transaction with CSL Behring as well as achieving milestones and royalties as defined in the CSL Behring Agreement;
- the cost and timing of future commercialization activities, including product manufacturing, marketing, sales and distribution of any of our product candidates for which we receive marketing approval in the future;
- the amount and timing of revenue, if any, we receive from commercial sales of any product candidates for which we, or our collaboration partner, receives marketing approval in the future;
- the scope, timing, results and costs of our current and planned clinical trials, including those for etranacogene dezaparvovec in hemophilia B and AMT-130 in Huntington's disease;
- the scope, timing, results and costs of preclinical development and laboratory testing of our additional product candidates;
- the need for additional resources and related recruitment costs to support the preclinical and clinical development of our product candidates;
- 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;
- our ability to enter into collaboration arrangements 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 January 2022 and will run through June 2023;
- 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 increasing the scale and capacity of our manufacturing capabilities; and
- the costs associated in preparing for the Biologics License Application ("BLA") submission of etranacogene dezaparvovec, including process validation, inspection readiness and other regulatory expenses in the event that our collaboration and license agreement with CSL Behring would not close.

Contractual obligations and commitments

The table below sets forth our contractual obligations and commercial commitments as of June 30, 2020, that are expected to have an impact on liquidity and cash flows in future periods.

	Less than 1 year	Between 1 and 2 years	Between 2 and 5 years	Over 5 years	Total
			(in thousands)		
Debt obligations (including $9.0 million interest payments)	$ 3,141	$ 14,076	$ 26,770	$ —	$ 43,987
Operating lease obligations	5,591	5,485	17,744	31,253	60,073
Total	**$ 8,732**	**$ 19,561**	**$ 44,514**	**$ 31,253**	**$ 104,060**

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 will also have obligations to make future payments that become due and payable if we collect the upfront payment or milestone payments from CSL Behring. We have not included these commitments on our balance sheet or in the table above because these payments only become due and payable upon the closing of the transaction with CSL Behring.

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.

Off-Balance Sheet Arrangements

As of June 30, 2020, 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, 2020, have 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, 2019, which was filed with the SEC on March 2, 2020.

Item 4. Controls and Procedures

Evaluation of Disclosure Controls and Procedures

Our management, with the participation of our chief executive and chief financial 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, 2020. Based on such evaluation, our CEO has concluded that as of June 30, 2020, 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 second quarter of 2020, 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 materially affect, our internal control over financial reporting, with the exception of changing the frequency of our controls related to the assessment of the realizability of net deferred tax assets from annual to quarterly. We have not experienced any material impact to our internal controls over financial reporting despite the fact that a large group of our employees are working remotely due to the COVID-19 pandemic. We are continually monitoring and assessing the impact the COVID-19 situation has on the operating effectiveness of our internal controls.

Part II – OTHER INFORMATION

Item 1. Legal Proceedings

None.

Item 1A. Risk Factors

An investment in our ordinary shares involves a high degree of risk. You should carefully consider the following information about these risks, together with the other information appearing elsewhere in this Quarterly Report on Form 10-Q, including our financial statements and related notes thereto, and the risk factors discussed in Part I, Item 1A "Risk Factors" in our Annual Report on Form 10-K for the year ended December 31, 2019, filed with the SEC on March 2, 2020, before deciding to invest in our ordinary shares. We operate in a dynamic and rapidly changing industry that involves numerous risks and uncertainties. The risks and uncertainties described below are not the only ones we face. Other risks and uncertainties, including those that we do not currently consider material, may impair our business. If any of the risks discussed below actually occur, our business, financial condition, operating results or cash flows could be materially adversely affected. This could cause the value of our securities to decline, and you may lose all or part of your investment.

Risks Related to the CSL Behring Collaboration and License Transaction

In June 2020, uniQure biopharma BV, our wholly-owned subsidiary, entered into a commercialization and license agreement (the "CSL Behring Agreement") with CSL Behring LLC ("CSL Behring") providing CSL Behring exclusive global rights to etranacogene dezaparvovec, our investigational gene therapy for patients with hemophilia B.

We and CSL Behring may be unable to close the transaction, and any delay in completing the transaction could diminish the anticipated benefits of the transaction or result in increased costs. Failure to close the transaction could adversely impact the market price of our shares as well as our business and operating results, cash flows and results of operations.

The closing of the transaction is contingent on completion of the successful reviews under antitrust laws in the United States, Australia, and the United Kingdom, including the expiration of the waiting period under the Hart-Scott-Rodino Antitrust Improvements Act of 1976, as amended. We cannot make any assurances that the transaction will be closed on the timeline currently contemplated or at all, or that, as part of the regulatory review process, that additional conditions or terms may be required.

While we and CSL Behring intend to pursue vigorously all required governmental clearances, the requirement to receive these clearances before the closing of the transaction could delay the transaction or result in an inability to complete the transaction. Any delay in the completion of the transaction could diminish anticipated benefits of the transaction, including realization of expected benefits of partnering, result in additional transaction costs, loss of revenue or other effects associated with uncertainty about the transaction. Any such delay could also delay the timelines associated with our commercialization of etranacogene dezaparvovec, including the filing of a biologics licensing application with the FDA, and such delays could cause us to bring etranacogene dezaparvovec to market after a competitive product in the United States, Europe or in other markets.

We will need to fund the investments into the development and preparation of the commercial launch of etranacogene dezaparvovec for as long as the regulatory reviews continue. The completions of these reviews could require significant time and/or might result in modifications or even denial of the transactions. These factors could adversely impact the cash flows and results that we are able to generate in relation to etranacogene dezaparvovec.

To the extent that the market price of the ordinary shares of our common stock reflects positive market assumptions that the transaction will close or that the transaction is advantageous to us, the price of such shares may decline if the transaction does not close for any reason or in a timely manner.

The announcement and pendency of the transaction with CSL Behring could adversely affect our business and operations.

Whether the transaction is ultimately closed or not, its announcement and pendency could have a number of negative effects on our current business, including potentially disrupting our regular operations, diverting the attention of our workforce and management team, or increasing workforce turnover. The completion of the transaction, including, for example, efforts to obtain regulatory clearances, may require significant time and attention from our management and divert attention from the day-to-day operations of our business. Any uncertainty over the ability of us and CSL Behring to complete the transaction could make it more difficult for us to retain certain key employees or attract new talent or to pursue business strategies.

Parties with which we have business relationships related to etranacogene dezaparvovec, either contractual or operational, may experience uncertainty as to the future or desirability of such relationships and may delay or defer certain business decisions, seek alternative relationships with third parties or seek to alter their present business relationships with us. Parties with whom we otherwise may have sought to establish business relationships may be reluctant to enter into agreements with us.

Risks Related to the Current COVID-19 Pandemic

A pandemic, epidemic or outbreak of an infectious disease, such as COVID-19, may materially and adversely affect our business and operations.

The recent outbreak of COVID-19 originated in Wuhan, China, in December 2019 and has since spread to multiple countries, including the United States and the Netherlands. On March 11, 2020, the WHO declared the outbreak a pandemic. The COVID-19 pandemic is affecting the United States and global economies and has affected and may continue to affect our operations and those of third parties on which we rely. The COVID-19 pandemic may cause disruptions in our raw material supply, our commercial-scale manufacturing capabilities for AAV-based gene therapies, the commercialization of our product candidates, and the conduct of current and future clinical trials. In addition, the COVID-19 pandemic has affected and may continue to affect the operations of the FDA, EMA and other health authorities, which could result in delays of reviews and approvals, including with respect to our product candidates. As evidenced by the postponement of procedures for two patients in our Phase I/II clinical study of AMT-130, the evolving COVID-19 pandemic has impacted the pace of enrollment and procedures in our clinical trials, as well as caused challenges in scheduling follow-up visits and managing other aspects of our clinical trials. We may be affected by similar delays as patients may avoid or may not be able to travel to healthcare facilities and physicians' offices unless due to a health emergency and clinical trial staff can no longer get to the clinic. Such facilities and offices have been and may continue to be required to focus limited resources on non-clinical trial matters, including treatment of COVID-19 patients, thereby decreasing availability, in whole or in part, for clinical trial services. In addition, employee disruptions and remote working environments related to the COVID-19 pandemic and the federal, state and local responses to such virus, has impacted and could continue to impact the efficiency and pace with which we work and develop our product candidates and our manufacturing capabilities. Further, while the potential economic impact brought by, and the duration of, the COVID-19 pandemic is difficult to assess or predict, the impact of the COVID-19 pandemic on the global financial markets may reduce our ability to access capital, which could negatively impact our short-term and long-term liquidity. Additionally, the stock market has been unusually volatile during the COVID-19 outbreak and such volatility may continue. To date, during certain periods of the COVID-19 pandemic, our stock price fluctuated significantly, and such fluctuation may continue to occur. The ultimate impact of the COVID-19 pandemic is highly uncertain and subject to change. We do not yet know the full extent of potential delays or impacts on our business, financing or clinical trial activities or on healthcare systems or the global economy as a whole. However, these effects could have a material impact on our liquidity, capital resources, operations and business and those of the third parties on which we rely.

Risks Related to the Development of Our Product Candidates

None of our product candidates have been approved for commercial sale and they might never receive regulatory approval or become commercially viable. We have never generated any significant revenue from product sales and may never be profitable.

All of our product candidates are in research or development. We have not generated any significant revenues from the sale of products or manufacturing of our product for a licensee and do not expect to generate any such revenue before 2022. Our lead product candidates, etranacogene dezaparvovec (also known as AMT-061) and AMT-130, and any of our other potential product candidates will require extensive preclinical and/or clinical testing and regulatory approval prior to commercial use. Our research and development efforts may not be successful. Even if our clinical development efforts result in positive data, our product candidates may not receive regulatory approval or be successfully introduced and marketed at prices that would permit us to operate profitably.

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 different stages of 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 clinical research organizations (“CROs”) and clinical trial sites;
- delays in receiving regulatory authorization to conduct the clinical trials or a regulatory authority decision that the clinical trial should not proceed;
- delays in obtaining required 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 Practice or applicable regulatory guidelines in other countries;
- difficulty or delays in patient recruiting into clinical trials;
- the impact of the COVID-19 pandemic on the healthcare system or any clinical trial sites;
- 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, clinical efficacy and safety, 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 clinical trial sites. Clinical trial sites may not have the adequate infrastructure established to handle gene therapy products or may have difficulty finding eligible patients to enroll into a trial.

In addition, we or any collaborators we may have may not be able to locate and enroll enough eligible patients to participate in these trials as required by the FDA, the EMA or similar regulatory authorities outside the United States and the European Union. This may result in our failure to initiate or continue clinical trials for our product candidates or may cause us to abandon one or more clinical trials altogether. Because our programs are focused on the treatment of patients with rare or orphan or ultra-orphan diseases, our ability to enroll eligible patients in these trials may be limited or slower than we anticipate considering the small patient populations involved and the specific age range required for treatment eligibility in some indications. In addition, our potential competitors, including major pharmaceutical, specialty pharmaceutical and biotechnology companies, academic institutions and governmental agencies and public and private research institutions, may seek to develop competing therapies, which would further limit the small patient pool available for our studies. Also, patients may be reluctant to enroll in gene therapy trial where there are other therapeutic alternatives available or that may become available, which may be for various reasons including uncertainty about the safety or effectiveness of the therapeutic and the possibility that treatment with the therapeutic would preclude future gene therapy treatments.

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 materially harm our business, financial conditions 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.

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. In 2017, we announced our plans to advance etranacogene dezaparvovec, which includes an AAV5 vector carrying the FIX-Padua transgene, into a pivotal study. While we believe etranacogene dezaparvovec and AMT-060, our product candidate that was previously studied in a Phase I/II study, have been demonstrated to be materially comparable in nonclinical studies and manufacturing quality assessments, it is possible that ongoing or future clinical studies of etranacogene dezaparvovec may show unexpected differences from AMT-060. Should these differences have an unfavorable impact on clinical outcomes, they may adversely impact our ability to achieve regulatory approval or market acceptance of etranacogene dezaparvovec.

In our Phase I/II clinical study of AMT-060, we screened patients for pre-existing anti-AAV5 antibodies to determine their eligibility for the trial. Three of the ten patients screened for the study tested positive for anti-AAV5 antibodies on reanalysis using a more sensitive antibody assay. Since we did not observe any ill-effects or correlation between the level of anti-AAV5 antibodies and clinical outcomes, patients who have anti-AAV5 antibodies are permitted to enroll in our planned pivotal study of etranacogene dezaparvovec. Since we only have been able to test a limited number of patients and have limited clinical and pre-clinical data, it is possible that ongoing or future clinical studies may not confirm these results, and if so, negatively impact the outcome of our study.

In advance of treating patients in the pivotal study of etranacogene dezaparvovec, we conducted a short study to confirm the dose expected to be used in the pivotal trial. The dose-confirmation study enrolled three patients, who were administered a single dose of $2x10^{13}$ gc/kg. We have relied on the short-term data from this study, including FIX activity and safety outcomes during the weeks following administration of etranacogene dezaparvovec, to confirm the dose to be used in the pivotal study. Following the results of this study, our Data Monitoring Committee confirmed the dose of $2x10^{13}$ gc/kg for administration in the pivotal study. Given the limited number of patients and short follow-up period, data from this study may differ materially from the future results of our planned pivotal study of etranacogene dezaparvovec.

A number of companies in the pharmaceutical and biotechnology industries have suffered significant setbacks in later-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 product candidates may not receive approval from the FDA or EMA. Data obtained from preclinical and clinical activities are subject to varying interpretations, which may delay, limit or prevent regulatory approval. In addition, we may encounter regulatory delays or rejections because of many factors, including changes in regulatory policy during the period of product development. Failure to confirm favorable results from earlier trials by demonstrating the safety and effectiveness of our products in later-stage clinical trials with larger patient populations could have a material adverse effect on our business, financial condition and results of operations.

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

We have obtained and may in the future seek one or more of fast track designation, breakthrough therapy designation, RMAT designation, PRIME scheme access or priority review designation for our product candidates. 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 and 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, where preliminary clinical evidence indicates that the drug may demonstrate substantial improvement over existing therapies on one or more clinically significant endpoints, such as substantial treatment effects observed early in clinical development. A RMAT designation is designed to accelerate approval for regenerative advanced therapies. Priority review designation is intended to speed the FDA marketing application review timeframe for drugs that treat a serious condition and, if approved, would provide a significant improvement in safety or effectiveness. PRIME is a scheme provided by the EMA, similar to the FDA's breakthrough therapy designation, 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 scheme, interaction and communication between the regulatory agency and the sponsor of the trial can help to identify the most efficient path for clinical development. Sponsors of drugs with fast track products or breakthrough therapies may also be able to submit marketing applications on a rolling basis, meaning that the FDA may review portions of a marketing application before the sponsor submits the complete application to the FDA, if the sponsor pays the user fee upon submission of the first portion of the marketing application. For products that receive a priority review designation, the FDA's marketing application review goal is shortened to six months, as opposed to ten to twelve months under standard review. RMAT designations may also expedite product candidate development and approval.

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

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

An element of our strategy is to use our gene therapy technology platform to expand our product pipeline and to progress these candidates through preclinical and clinical development ourselves or together with collaborators. Although we currently have a pipeline of programs at various stages of development, we may not be able to identify or develop product candidates that are safe and effective. Even if we are successful in continuing to build our pipeline, the potential product candidates that we identify may not be suitable for clinical development. Research programs to identify new product candidates require substantial technical, financial and human resources. We or any collaborators may focus our efforts and resources on potential programs or product candidates that ultimately prove to be unsuccessful. If we do not continue to successfully develop and commercialize product candidates based upon our technology, we may face difficulty in obtaining product revenues in future periods, which could result in significant harm to our business, results of operations and financial position and materially 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 from time to time in part by in-licensing the rights to key technologies, including those related to gene delivery, genes and gene cassettes. The future growth of our business will depend in significant part on our ability to in-license or otherwise acquire the rights to additional product candidates or technologies, particularly through our collaborations with academic research institutions. However, we may be unable to in-license or acquire the rights to any such product candidates or technologies from third parties on acceptable terms or at all. The in-licensing and acquisition of these technologies is a competitive area, and many more established companies are also pursuing strategies to license or acquire product candidates or technologies that we may consider attractive. These established companies may have a competitive advantage over us due to their size, cash resources and greater clinical development and commercialization capabilities. In addition, companies that perceive us to be competitors may be unwilling to license rights to us. Furthermore, we may be unable to identify suitable product candidates or technologies within our areas of focus. If we are unable to successfully obtain rights to suitable product candidates or technologies, our business, financial condition and prospects could suffer.

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

Public perception may be influenced by claims that gene therapy is unsafe, and gene therapy may not gain the acceptance of the public or the medical community. The risk of cancer remains a concern for gene therapy, and we cannot assure that it will not occur in any of our planned or future clinical studies. In addition, there is the potential risk of delayed adverse events following exposure to gene therapy products due to persistent biological activity of the genetic material or other components of products used to carry the genetic material.

As of June 30, 2020, a total of three patients reported serious adverse events related to the treatment of AMT-060, our first generation hemophilia B gene therapy, in our Phase I/II 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. Additionally, one patient in our ongoing Phase IIb study of etranacogene dezaparvovec underwent hip surgery due to a pre-existing condition and was treated perioperatively with short-acting factor replacement. This was reported by the investigator as a serious adverse event unrelated to etranacogene dezaparvovec.

Adverse events in our clinical trials or those conducted by other parties (even if not ultimately attributable to our product candidates), and the resulting publicity, could result in increased governmental regulation, unfavorable public perception, failure of the medical community to accept and prescribe gene therapy treatments, potential regulatory delays in the testing or approval of our product candidates, stricter labeling requirements for those product candidates that are approved and a decrease in demand for any such product candidates. If any of these events should occur, it may have a material adverse effect on our business, financial condition and results of operations.

Risks Related to Our Manufacturing

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

Our manufacturing facility in Lexington is subject to ongoing regulation and periodic inspection by the FDA, EMA 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 FDA, EMA, 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, financial condition and results of operations.

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

The insect-cell based manufacturing process we use to produce our products and product candidates is highly complex and in the normal course is subject to variation or production difficulties. Issues with any of our manufacturing processes, even minor deviations from the normal process, could result in insufficient yield, product deficiencies or manufacturing failures that result in adverse patient reactions, lot failures, insufficient inventory, product recalls and product liability claims. Additionally, we may not be able to scale up some or all of our manufacturing processes that may results in delays in regulatory approvals or otherwise adversely affect our ability to manufacture sufficient amounts of our products.

Many factors common to the manufacturing of most biologics and drugs could also cause production interruptions, including raw materials shortages, raw material failures, growth media failures, equipment malfunctions, facility contamination, labor problems, natural disasters, disruption in utility services, terrorist activities, or cases of force majeure and acts of god (including the effects of the COVID-19 pandemic) beyond our control. We also may encounter problems in hiring and retaining the experienced specialized personnel needed to operate our manufacturing process, which could result in delays in our production or difficulties in maintaining compliance with applicable regulatory requirements.

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

Our use of viruses, chemicals and other hazardous materials requires us to comply with regulatory requirements and exposes us to significant potential liabilities.

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

Our resources might be adversely affected if we are unable to meet our product supply needs and obligations.

To meet our expected future production needs, we will need to complete the validation of our existing manufacturing processes as well as to develop larger scale manufacturing processes. We might be unable to successfully complete the validation or development to sufficiently meet our future production needs. As a result, we may need to dedicate more of our resources to complete the validation or development, which could adversely impact our ability to develop our other proprietary programs, to meet our production needs, to conserve our cash, or to receive financial payments pursuant to our agreement with third parties, including with CSL Behring in return for supplying etranacogene dezaparvovec following regulatory approval.

Risks Related to Regulatory Approval of Our Products

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

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

We believe that all our current product candidates will be viewed as gene therapy products by the applicable regulatory authorities. While there are a number of gene therapy product candidates under development, in the United States, FDA has only approved a limited number of gene therapy products, to date. Accordingly, regulators, like FDA, may have limited experience with the review and approval of marketing applications for gene therapy products.

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 continue to evolve, 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. In the United States, there have been a number of recent changes relating to gene therapy development. By example, FDA issued a number of new guidance documents on human gene therapy development, one of which was specific to human gene therapy for hemophilia and another of which was specific to rare diseases. Moreover, the U.S. National Institutes of Health, which also has authority over research involving gene therapy products, issued a proposed rule in October 2018, seeking to streamline the oversight of such protocols and reduce duplicative reporting requirements that are already captured within existing regulatory frameworks. Moreover, the European Commission conducted a public consultation in early 2013 on the application of EU legislation that governs advanced therapy medicinal products, including gene therapy products, which could result in changes in the data we need to submit to the EMA for our product candidates to gain regulatory approval or change the requirements for tracking, handling and distribution of the products which may be associated with increased costs. In addition, divergent scientific opinions among the various bodies involved in the review process may result in delays, require additional resources and ultimately result in rejection. The FDA, EMA, and other regulatory authorities will likely continue to revise and further update its approach to gene therapies in the coming years. These regulatory agencies, committees and advisory groups and the new regulations and guidelines they promulgate may lengthen the regulatory review process, require us to perform additional studies, increase our development costs, lead to changes in regulatory positions and interpretations, delay or prevent approval and commercialization of our product candidates or lead to significant post-approval limitations or restrictions. Delay or failure to obtain, or unexpected costs in obtaining, the regulatory approval necessary to bring a potential product to market could decrease our ability to generate sufficient product revenues to maintain our business.

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

Regulatory authorities in some jurisdictions, including the United States and the European Union, 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 FDA or EMA from approving another marketing application for the same drug for the same indication for that period. The FDA and EMA, however, may subsequently approve a similar drug for the same indication during the first product's market exclusivity if the FDA or 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 FDA or EMA 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. The inability to obtain or failure to maintain adequate product exclusivity for our product candidates could have a material adverse effect on our business prospects, results of operations and financial conditions.

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

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

- closing and successful execution of our transaction with CSL Behring for the commercialization of etranacogene dezaparvovec;
- successful completion of preclinical studies and clinical trials, and other work required by regulators;
- 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 approvals using our manufacturing facility in Lexington, Massachusetts;
- launch and commercialization of our products, if approved, whether alone or in collaboration with others;
- identifying and engaging effective distributors or resellers on acceptable terms in jurisdictions where we plan to utilize third parties for the marketing and sales of our product candidates;
- acceptance of our products, if approved, by patients, the medical community and third-party payers;
- effectively competing with existing therapies and gene therapies based on safety and efficacy profile;
- achieve optimal pricing based on durability of expression, safety and efficacy;
- obtaining and maintaining healthcare coverage and adequate reimbursement;
- complying with any applicable post-approval requirements and maintaining a continued acceptable overall safety profile; and
- obtaining adequate reimbursement for the total patient population and each subgroup to sustain a viable commercial business model in U.S. and EU markets.

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

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

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

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, reimbursement may not be sufficient to sustain a viable business for all sub populations being studied, 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 markets for AAV-based gene therapies may be 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 may not be eligible for administration of a gene therapy that includes this particular capsid. For example, etranacogene dezaparvovec, our gene therapy candidate for hemophilia B patients, incorporates an AAV5 capsid. In our Phase I/II clinical study of AMT-060, we screened patients for pre-existing anti-AAV5 antibodies to determine their eligibility for the trial. Three of the ten patients screened for the study tested positive for anti-AAV5 antibodies on reanalysis. However, we did not observe any ill-effects or correlation between the level of anti-AAV5 antibodies and clinical outcomes in these three patients, suggesting that patients who have anti-AAV5 antibodies may still be eligible for AAV5-based gene therapies. Since we only have been able to test a limited number of patients and have limited clinical and pre-clinical data, it is possible that future clinical studies may not confirm these results. This may limit the addressable market for etranacogene dezaparvovec and any future revenues derived from the sale of the product, if approved.

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 treatments. The degree of market acceptance of any of our product candidates that receive marketing approval in the future will depend on many factors, including:

- the efficacy and potential advantages of our therapies compared with alternative treatments;
- our ability to convince payers of the long-term cost-effectiveness of our therapies and, consequently, the availability of third-party coverage and adequate reimbursement;
- the cost of treatment with gene therapies, including ours, in comparison to traditional chemical and small-molecule treatments;
- 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 by regulators on the use of our products.

A failure to gain market acceptance for any of the above reasons, or any reasons at all, by a gene therapy for which we receive regulatory approval would likely hinder our ability to recapture our substantial investments in that and other gene therapies and could have a material adverse effect on our business, financial condition and results of operation.

If we are unable to expand our commercialization capabilities or enter into agreements with third parties to market and sell any of our product candidates for which we obtain marketing approval, we may be unable to generate any product revenue.

To successfully commercialize any products that may result from our development programs, we need to continue to expand our commercialization capabilities, either on our own or with others. The development of our own market development effort is, and will continue to be, expensive and time-consuming and could delay any product launch. Moreover, we cannot be certain that we will be able to successfully develop this capability.

We may enter into collaborations regarding our other product candidates with other entities to utilize their established marketing and distribution capabilities, but we may be unable to enter into such agreements on favorable terms, if at all. If any current or future collaborators do not commit sufficient resources to commercialize our products, or we are unable to develop the necessary capabilities on our own, we will be unable to generate sufficient product revenue to sustain our business. We compete with many companies that currently have extensive, experienced and well-funded medical affairs, marketing and sales operations to recruit, hire, train and retain marketing and sales personnel. We also may face competition in any search for third parties to assist us with the sales and marketing efforts of our product candidates. Without an internal team or the support of a third party to perform marketing and sales functions, we may be unable to compete successfully against these more established companies.

If the market opportunities for our product candidates are smaller than we believe they are, our product revenues may be adversely affected, and our business may suffer.

We focus our research and product development on treatments for severe genetic and orphan diseases. Our understanding of both the number of people who have these diseases, as well as the subset of people with these diseases who have the potential to benefit from treatment with our product candidates, are based on estimates. These estimates may prove to be incorrect and new studies may reduce the estimated incidence or prevalence of these diseases. The number of patients in the United States, the EU and elsewhere may turn out to be lower than expected, may not be otherwise amenable to treatment with our products or patients may become increasingly difficult to identify and access, all of which would adversely affect our business, financial condition, results of operations and prospects.

Further, there are several factors that could contribute to making the actual number of patients who receive other potential products less than the potentially addressable market. These include the lack of widespread availability of, and limited reimbursement for, new therapies in many underdeveloped markets. Further, the severity of the progression of a disease up to the time of treatment, especially in certain degenerative conditions, could diminish the therapeutic benefit conferred by a gene therapy. Lastly, certain patients' immune systems might prohibit the successful delivery of certain gene therapy products to the target tissue, thereby limiting the treatment outcomes.

Our gene therapy approach utilizes vectors derived from viruses, which may be perceived as unsafe or may result in unforeseen adverse events. Negative public opinion and increased regulatory scrutiny of gene therapy may damage public perception of the safety of our product and product candidates and adversely affect our ability to conduct our business or obtain regulatory approvals for our product candidates.

Gene therapy remains a novel technology. 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. In particular, our success will depend upon physicians who specialize in the treatment of genetic diseases targeted by our product and product candidates, if approved, prescribing treatments that involve the use of our product and product candidates, if approved, in lieu of, or in addition to, existing treatments with which they are familiar and for which greater clinical data may be available. More restrictive government regulations or negative public opinion would have an adverse effect on our business, financial condition, results of operations and prospects and may delay or impair the development and commercialization of our product candidates or demand for any products we may develop. For example, earlier gene therapy trials led to several well-publicized adverse events, including cases of leukemia and death seen in other trials using other vectors. Serious adverse events in our clinical trials, or other clinical trials involving gene therapy products or our competitors' products, even if not ultimately attributable to the relevant product candidates, and the resulting publicity, could result in increased government regulation, unfavorable public perception, 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 products for which we obtain marketing approval.

Ethical, legal and social issues may reduce demand for any gene therapy products for which we obtain marketing approval.

Prior to receiving certain gene therapies, patients may be required to undergo genetic testing. Genetic testing has raised concerns regarding the appropriate utilization and the confidentiality of information provided by genetic testing. Genetic tests for assessing a person's likelihood of developing a chronic disease have focused public attention on the need to protect the privacy of genetic information. For example, concerns have been expressed that insurance carriers and employers may use these tests to discriminate on the basis of genetic information, resulting in barriers to the acceptance of genetic tests by consumers. This could lead to governmental authorities restricting genetic testing or calling for limits on or regulating the use of genetic testing, particularly for diseases for which there is no known cure. Any of these scenarios could decrease demand for any products for which we obtain marketing approval.

If we obtain approval to commercialize any of our product candidates outside of the United States, a variety of risks associated with international operations could materially adversely affect our business.

We expect that we will be subject to additional risks in commercializing any of our product candidates outside the United States, including:

- different regulatory requirements for approval of drugs and biologics in foreign countries;
- reduced protection for intellectual property rights;
- unexpected changes in tariffs, trade barriers and regulatory requirements;
- economic weakness, including inflation, or political instability in particular foreign economies and markets;
- compliance with tax, employment, immigration and labor laws for employees living or traveling abroad;
- foreign currency fluctuations, which could result in increased operating expenses and reduced revenues, and other obligations incident to doing business in another country;
- workforce uncertainty in countries where labor unrest is more common than in the United States;
- production shortages resulting from any events affecting raw material supply or manufacturing capabilities abroad; and
- business interruptions resulting from geopolitical actions, including war and terrorism or natural disasters including earthquakes, typhoons, floods and fires.

We face substantial competition, and others may discover, develop or commercialize competing 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 intense 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 Applied Genetic Technologies Corp., Abeona Therapeutics, Adverum Biotechnologies, Allergan, Ally Therapeutics, Asklepios BioPharmaceutical, Astellas, AVROBIO, Axovant Gene Therapies, Bayer, Biogen, BioMarin, bluebird bio, CRISPR Therapeutics, Editas Medicine, Expression Therapeutics, Freeline Therapeutics, Generation Bio, Genethon, GlaxoSmithKline, Homology Medicines, Intellia Therapeutics, Johnson & Johnson, Krystal Biotech, LogicBio Therapeutics, Lysogene, MeiraGTx, Milo Biotechnology, Mustang Bio, Novartis, Orchard Therapeutics, Oxford Biomedica, Pfizer, REGENXBIO, Renova Therapeutics, Roche, Rocket Pharmaceuticals, Sangamo Therapeutics, Sanofi, Selecta Biosciences, Sarepta Therapeutics, Solid Biosciences, Takeda, Ultragenyx, Vivet Therapeutics, and Voyager Therapeutics, as well as several companies addressing other methods for modifying genes and regulating gene expression. We may also face competition with respect to the treatment of some of the diseases that we are seeking to target with our gene therapies from protein, nucleic acid, antisense, RNAi and other pharmaceuticals under development or commercialized at pharmaceutical and biotechnology companies such as Alnylam Pharmaceuticals, Amgen, Bayer, Biogen, BioMarin, CSL Behring, Dicerna Pharmaceuticals, Ionis Pharmaceuticals, Novartis, Novo Nordisk, Pfizer, Translate Bio, Roche, Sanofi, Sobi, Takeda, WaVe Life Sciences, 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.

If we do not achieve our projected development goals in the timeframes we announce and expect, the commercialization of our product candidates may be delayed and, as a result, our stock price may decline.

For planning purposes, we estimate the timing of the accomplishment of various scientific, clinical, regulatory and other product development goals, or development milestones. These development milestones may include the commencement or completion of scientific studies, clinical trials, the submission of regulatory filings, and approval for commercial sale. From time to time, we publicly announce the expected timing of some of these milestones. All of these milestones are based on a variety of assumptions. The actual timing of these milestones can vary dramatically compared to our estimates, in many cases for reasons beyond our control. If we do not meet these milestones, including those that are publicly announced, the commercialization of our products may be delayed and, as a result, our stock price may decline.

Risks Related to Our Dependence on Third Parties

Our ongoing discussions with BMS to restructure or amend the terms of our collaboration may not be successful or may result in material changes to these arrangements.

The research term of our collaboration and license agreement with BMS expired in May 2019, and we are currently in discussions with BMS potentially to restructure or amend that agreement and the other related agreements to eliminate, reduce or alter our obligations under the collaboration. Our discussions are ongoing and may or may not result in any restructuring or changes to our collaboration. If a restructuring of our collaboration with BMS were to be concluded, we expect it would result in a termination or amendment of existing agreements, or the execution of new agreements that collectively could include changes in the number of future collaboration targets that may be designated by BMS, the exclusivity provisions related to collaboration targets, our obligations to provide manufacturing services for collaboration targets, as well as changes in or the elimination of our economic rights on collaboration targets, milestone payments, and BMS's warrants to purchase our ordinary shares, among other potential matters. Any such restructuring, if concluded, may include additional or different provisions from those described above, and may include economic or other terms that are less advantageous for us.

Because the outcome of these discussions is unknown, we have not taken into account the impact of such restructuring, if any, on the timing of recognizing prepaid license revenue, or any other potential financial metrics, in our consolidated financial statements. We will account for any potential changes if and when the agreements are restructured or amended.

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 enter into or maintain key collaboration or other contractual arrangements, our business could be adversely affected.

We have in the past 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 development programs.

Any collaboration, 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 may have limited or no control over the design or conduct of clinical trials sponsored by collaborators;
- we may be hampered from entering into collaboration arrangements if we are unable to obtain consent from our licensors to enter into sublicensing arrangements of technology we have in-licensed;
- if any collaborator does 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, for instance, 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 any collaboration does not result in the successful development and commercialization of products or if a collaborator were to terminate an agreement with us, we may not receive future research funding or milestone or royalty payments under that collaboration, and we may lose access to important technologies and capabilities of the collaboration. All the risks relating to product development, regulatory approval and commercialization described herein also apply to the activities of any 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.

Our licensing arrangements with third parties may 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 either in part or in whole, 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, in part, upon a combination of forms of intellectual property, including in-licensed and owned patents to protect our intellectual property. Our success depends in a large part on our ability to obtain and maintain this protection in the United States, the European Union, 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. For example, patents we own currently are and may become subject to future patent opposition or similar proceedings, which may result in loss of scope of some claims or the entire patent. Our competitors may be able to circumvent our owned or licensed patents by developing similar or alternative technologies or products in a non-infringing manner.

Successful challenges to our patents may result in loss of exclusivity or freedom to operate or in patent claims being narrowed, invalidated or held unenforceable, in whole or in part, which could limit our ability to stop others from using or commercializing similar or identical technology and products, or limit the duration of the patent protection of our technology and products.

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

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

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

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

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

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

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

If we are unable to protect the confidentiality of our proprietary information and know-how, the value of our technology and products could be adversely affected.

In addition to seeking patent protection, we also rely on other proprietary rights, including protection of trade secrets, know-how and confidential and proprietary information. To maintain the confidentiality of our trade secrets and proprietary information, we enter into confidentiality agreements with our employees, consultants, collaborators and other third parties who have access to our trade secrets. Our agreements with employees also provide that any inventions conceived by the individual in the course of rendering services to us will be our exclusive property. However, we may not obtain these agreements in all circumstances, and individuals with whom we have these agreements may not comply with their terms. The assignment of intellectual property rights may not be self-executing, or the assignment agreements may be breached, and we may be forced to bring claims against third parties, or defend claims that they may bring against us, to determine the ownership of what we regard as our intellectual property. In addition, in the event of unauthorized use or disclosure of our trade secrets or proprietary information, these agreements, even if obtained, may not provide meaningful protection, particularly for our trade secrets or other confidential information. To the extent that our employees, consultants or contractors use technology or know-how owned by third parties in their work for us, disputes may arise between us and those third parties as to the rights in related inventions.

Adequate remedies may not exist in the event of unauthorized use or disclosure of our confidential information including a breach of our confidentiality agreements. Enforcing a claim that a party illegally disclosed or misappropriated a trade secret is difficult, expensive, and time consuming, and the outcome is unpredictable. In addition, some courts in and outside of 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 or other third party, we would have no right to prevent them from using that technology or information to compete with us. The disclosure of our trade secrets or the independent development of our trade secrets by a competitor or other third party would impair our competitive position and may materially harm our business, financial condition, results of operations, stock price and prospects.

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

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

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

Risks Related to Pricing and Reimbursement

We face uncertainty related to insurance coverage of, and pricing and reimbursement for product candidates for which we may receive marketing approval.

We anticipate that the cost of treatment using our product candidates will be significant. We expect that most patients and their families will not be capable of paying for our products themselves. There will be no commercially viable market for our product candidates without reimbursement from third party payers, such as government health administration authorities, private health insurers and other organizations. Even if there is a commercially viable market, if the level of third-party reimbursement is below our expectations, most patients may not be able to afford treatment with our products and 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 for which medications they will pay and, subsequently, 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, pending or potential legislative and regulatory changes regarding the healthcare system and insurance coverage, could result in more rigorous coverage criteria and downward pressure on drug prices, and may affect our ability to profitably sell any products for which we obtain marketing approval.

The pricing review period and pricing negotiations for new medicines take considerable time and have uncertain results. Pricing review and negotiation usually begins only after the receipt of regulatory marketing approval, and some authorities require approval of the sale price of a product before it can be marketed. In some markets, particularly the countries of the European Union, prescription pharmaceutical pricing remains subject to continuing direct governmental control and to drug reimbursement programs even after initial approval is granted and price reductions may be imposed. Prices of medical products may also be subject to varying price control mechanisms or limitations as part of national health systems if products are considered not cost-effective or where a drug company's profits are deemed excessive. In addition, pricing and reimbursement decisions in certain countries can lead to mandatory price reductions or additional reimbursement restrictions in other countries. Because of these restrictions, any product candidates for which we may obtain marketing approval may be subject to price regulations that delay or prohibit our or our partners' commercial launch of the product in a particular jurisdiction. In addition, we or any collaborator may elect to reduce the price of our products to increase the likelihood of obtaining reimbursement approvals. If countries impose prices, which are not sufficient to allow us or any collaborator to generate a profit, we or any collaborator may refuse to launch the product in such countries or withdraw the product from the market. If pricing is set at unsatisfactory levels, or if the price decreases, our business could be harmed, possibly materially. If we fail to obtain and sustain an adequate level of coverage and reimbursement for our products by third party payers, our ability to market and sell our products would be adversely affected and our business would be harmed.

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

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

We also anticipate that many or all of our gene therapy product candidates may provide long-term, and potentially curative benefit, with a single administration. This is a different paradigm than that of other pharmaceutical therapies, which often require an extended course of treatment or frequent administration. As a result, governments and other payers may be reluctant to provide the significant level of reimbursement that we seek at the time of administration of our gene therapies or may seek to tie reimbursement to clinical evidence of continuing therapeutic benefit over time. Although it is possible that our product candidates will need to be administered only once, there may be situations in which re-administration is required, which may further complicate the pricing and reimbursement for these treatments. In addition, considering the anticipated cost of these therapies, governments and other payers may be particularly restrictive in making coverage decisions. These factors could limit our commercial success and materially 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 $70.6 million in the six months ended June 30, 2020, $124.2 million in the full year 2019 and $83.3 million in the full year 2018. As of June 30, 2020, we had an accumulated deficit of $730.3 million. To date, we have financed our operations primarily through the sale of equity securities and convertible debt, venture loans, through upfront payments from our collaboration partners and, to a lesser extent, subsidies and grants from governmental agencies and fees for services. We have devoted substantially all our financial resources and efforts to research and development, including preclinical studies and clinical trials. 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. Our losses will be materially impacted by the amount of license revenue that we will recognize in accordance with ASC 606 following the potential closing of our collaboration and license agreement with CSL Behring.

We anticipate that our expenses will increase substantially as we:

- Advance the clinical development of AMT-130, our Huntington's disease gene therapy program;
- Build-out our commercial and medical affairs infrastructure and seek marketing approval for any product candidates (including etranacogene dezaparvovec in case our collaboration and license agreement with CSL Behring would not close) that successfully complete clinical trials;
- Advance multiple research programs related to gene therapy candidates targeting liver-directed and CNS diseases;
- Continue to expand, enhance and optimize our technology platform, including our manufacturing capabilities, next-generation viral vectors and promoters, and other enabling technologies;
- Continue to expand our employee base to support research and development, as well as general and administrative functions;
- Acquire or in-license rights to new therapeutic targets or product candidates; and
- Maintain, expand and protect our intellectual property portfolio, including in-licensing additional intellectual property rights from third parties.

We 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 particular if the CSL Behring transaction would not close. 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 Second Amended and Restated Loan and Security Agreement (as amended, the "2018 Amended Facility") with Hercules Technology Growth Capital, Inc. ("Hercules") and our pledge to Hercules of substantially all our assets as collateral. If we raise additional capital through the sale of equity or convertible debt securities, our shareholders' ownership interest will be diluted, and the terms of these securities may include liquidation or other preferences that adversely affect the rights of holders of our ordinary shares.

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

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

As of June 30, 2020, we had $35.0 million of outstanding principal of borrowings under the 2018 Amended Facility, which we are required to repay in monthly principal installments from January 2022 through June 2023. 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 the 2018 Amended Facility, the occurrence of an event that would reasonably be expected to have a material adverse effect on our business, operations, assets or condition is an event of default. If an event of default occurs and the lender accelerates the amounts due, we may not be able to make accelerated payments, and the lender could seek to enforce security interests in the collateral securing such indebtedness, which includes substantially all our assets.

Risks Related to Other Legal Compliance Matters

Our relationships with customers and third-party payers will be subject to applicable anti-kickback, anti-bribery, fraud and abuse and other laws and regulations, which, if we are found in violation thereof, 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 would be able to 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. The cost associated with any of these actions could be substantial and could cause irreparable harm to our reputation or otherwise have a material adverse effect on our business, financial condition and results of operations.

We are subject to laws governing data protection in the different jurisdictions in which we operate. The implementation of such data protection regimes is complex, and should we fail to fully comply, we may be subject to penalties that they may have an adverse effect on our business, financial condition and results of operations.

Many national and state laws govern the privacy and security of health information and other personal and private information. They often differ from each other in significant ways. For instance, the EU has adopted a comprehensive data protection law called the General Data Protection Regulation ("GDPR") that took effect in May 2018. The GDPR, together with the national legislation of the EU member states governing the processing of personal data, impose strict obligations and restrictions on the ability to collect, analyze and transfer personal data, including health data from clinical trials and adverse event reporting. In particular, these obligations and restrictions concern the consent of the individuals to whom the personal data relates, the information provided to the individuals, the transfer of personal data out of the EU, security breach notifications, security and confidentiality of the personal data, and imposition of substantial potential fines for breaches of the data protection obligations. The GDPR imposes penalties for non-compliance of up to the greater of EUR 20 million or 4% of worldwide revenue. Data protection authorities from the different EU member states may interpret the GDPR and national laws differently and impose additional requirements, which add to the complexity of processing personal data in the EU. Guidance on implementation and compliance practices are often updated or otherwise revised. The significant costs of compliance with, risk of regulatory enforcement actions under, and other burdens imposed by the GDPR as well as under other regulatory schemes throughout the world related to privacy and security of health information and other personal and private data could have an adverse impact on our business, financial condition and results of operations.

If we fail to comply with environmental, health and safety laws and regulations, we could become subject to fines or penalties or incur costs that could harm our business.

We are subject to numerous environmental, health and safety laws and regulations, including those governing laboratory procedures and the handling, use, storage, treatment and disposal of hazardous materials and wastes. Our operations involve the use of hazardous and flammable materials, including chemicals and biological materials. Our operations also produce hazardous waste products. We cannot eliminate the risk of contamination or injury from these materials. We also could incur significant costs associated with civil or criminal fines and penalties for failure to comply with such laws and regulations.

Although we maintain employer's liability insurance to cover us for costs and expenses we may incur due to injuries to our employees resulting from the use of hazardous materials, this insurance may not provide adequate coverage against potential liabilities. We do not maintain insurance for environmental liability or toxic tort claims that may be asserted against us in connection with our storage or disposal of biological, hazardous or radioactive materials.

In addition, we may incur substantial costs to comply with current or future environmental, health and safety laws and regulations. These current or future laws and regulations may impair our research, development or production efforts. Our failure to comply with these laws and regulations also may result in substantial fines, penalties or other sanctions that could have a material adverse effect on our business, financial condition and results of operations.

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 coverages ranging from EUR 500,000 to EUR 6,500,000 per occurrence and per clinical trial. 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. In the event insurance coverage is insufficient to cover liabilities that we may incur, it could have a material adverse effect on our business, financial condition and results of operations.

Healthcare legislative and regulatory reform measures may have a material adverse effect on our financial operations.

Our industry is highly regulated and changes in law may adversely impact our business, operations or financial results. The Patient Protection and Affordable Care Act, as amended by the Health Care and Education Reconciliation Act, or the PPACA, is a sweeping measure intended to, among other things, expand healthcare coverage within the United States, primarily through the imposition of health insurance mandates on employers and individuals and expansion of the Medicaid program. Several provisions of the law may affect us and increase certain of our costs.

In addition, other legislative changes have been adopted since the PPACA was enacted. These changes include aggregate reductions in Medicare payments to providers of 2% per fiscal year, which went into effect on April 1, 2013 and, following passage of the Bipartisan Budget Act of 2018, will remain in effect through 2027 unless additional Congressional action is taken. In January 2013, President Obama signed into law the American Taxpayer Relief Act of 2012, which, among other things, further reduced Medicare payments to several types of providers and increased the statute of limitations period for the government to recover overpayments to providers from three to five years. These laws may result in additional reductions in Medicare and other healthcare funding, which could have a material adverse effect on our customers and, accordingly, our financial operations.

We anticipate that the PPACA, as well as other healthcare reform measures that may be adopted in the future, may result in more rigorous coverage criteria and an additional downward pressure on the reimbursement our customers may receive for our products. Further, there have been, and there may continue to be, judicial and Congressional challenges to certain aspects of the PPACA. For example, the U.S. Tax Cuts and Jobs Act of 2017 includes a provision repealing, effective January 1, 2019, the tax-based shared responsibility payment imposed by the Affordable Care Act on certain individuals who fail to maintain qualifying health coverage for all or part of a year that is commonly referred to as the "individual mandate". Additional legislative and regulatory changes to the PPACA, its implementing regulations and guidance and its policies, remain possible in the 116th U.S. Congress and under the Trump Administration. However, it remains unclear how any new legislation or regulation might affect the prices we may obtain for any of our product candidates for which regulatory approval is obtained. Any reduction in reimbursement from Medicare and other government programs may result in a similar reduction in payments from private payers. The implementation of cost containment measures or other healthcare reforms may prevent us from being able to generate revenue, attain profitability or commercialize our products.

Our internal computer systems, or those of our collaborators or other contractors or consultants, may fail or suffer security breaches, which could result in a material disruption of our product development programs.

Our internal computer systems and those of our current and any future collaborators and other contractors or consultants are vulnerable to damage from computer viruses, unauthorized access, natural disasters, terrorism, war and telecommunication and electrical failures. The size and complexity of our information technology systems, and those of our collaborators, contractors and consultants, and the large amounts of confidential information stored on those systems, make such systems vulnerable to service interruptions or to security breaches from inadvertent or intentional actions by our employees, third-party vendors and/or business partners, or from cyber-attacks by malicious third parties. Cyber-attacks are increasing in their frequency, sophistication and intensity, and have become increasingly difficult to detect. Cyber-attacks could include the deployment of harmful malware, ransomware, denial-of-service attacks, social engineering and other means to affect service reliability and threaten the confidentiality, integrity and availability of information. Cyber-attacks also could include phishing attempts or e-mail fraud to cause payments or information to be transmitted to an unintended recipient. The increased number of employees working remotely due to COVID-19 might increase our vulnerability to the above risk.

While we have not experienced a system failure, accident, cyber-attack, or security breach that has resulted in a material interruption in our operations to date, we have experienced and addressed recent system failures, cyber-attacks and security breaches. In the future, such events could result in a material disruption of our development programs and our business operations, whether due to a loss of our trade secrets or other proprietary information or other similar disruptions. Additionally, any such event that leads to unauthorized access, use or disclosure of personal information, including personal information regarding our patients or employees, could harm our reputation, cause us not to comply with federal and/or state breach notification laws and foreign law equivalents and otherwise subject us to liability under laws and regulations that protect the privacy and security of personal information. Security breaches and other inappropriate access can be difficult to detect, and any delay in identifying them may lead to increased harm of the type described above. While we have implemented security measures to protect our information technology systems and infrastructure, there can be no assurance that such measures will prevent service interruptions or security breaches that could adversely affect our business and the further development and commercialization of our product and product candidates could be delayed.

Risks Related to Employee Matters and Managing Growth

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

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

The loss of the services of our key employees could impede the achievement of our research and development objectives and seriously harm our ability to successfully implement our business strategy. Furthermore, replacing senior management and key employees may be difficult and may take an extended period because of the limited number of individuals in our industry with the breadth and depth of skills and experience required to successfully develop gene therapy products. Competition to hire from this limited pool is intense, and we may be unable to hire, train, retain or motivate these key personnel on acceptable terms.

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

Risks Related to Our Ordinary Shares

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

Our share price has been and may in the future be volatile. From the start of trading of our ordinary shares on the Nasdaq Global Select Market on February 4, 2014 through July 28, 2020, the sale price of our ordinary shares ranged from a high of $82.49 to a low of $4.72. The closing price on July 28, 2020, was $39.52 per ordinary share. The stock market in general and the market for smaller biopharmaceutical companies in particular, have experienced extreme volatility that has often been unrelated to the operating performance of particular companies. The market price for our ordinary shares may be influenced by many factors, including:

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

- general economic, industry and market conditions.

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

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

Our directors, 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, executive officers and major shareholders holding more than 5% of our outstanding ordinary shares, in the aggregate, beneficially own approximately 46.0% of our issued shares (including such shares to be issued in relation to exercisable options to purchase ordinary shares) as at June 30, 2020. As a result, if these shareholders were to choose to act together, they may be able, as a practical matter, to control many matters submitted to our shareholders for approval, as well as our management and affairs. For example, these persons, if they choose to act together, could control the election of the board directors and the approval of any merger, consolidation or sale of all or substantially all our assets. These shareholders may have interests that differ from those of other of our shareholders and conflicts of interest may arise.

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; and
- a requirement that certain matters, including an amendment of our articles of association, may only be brought to our shareholders for a vote upon a proposal by our board.

We do not expect to pay dividends in the foreseeable future.

We have not paid any dividends since our incorporation. Even if future operations lead to significant levels of distributable profits, we currently intend that earnings, if any, will be reinvested in our business and that dividends will not be paid until we have an established revenue stream to support continuing dividends. Accordingly, shareholders cannot rely on dividend income from our ordinary shares and any returns on an investment in our ordinary shares will likely depend entirely upon any future appreciation in the price of our ordinary shares.

If we fail to maintain an effective system of internal controls, we may be unable to accurately report our results of operations or prevent fraud or fail to meet our reporting obligations, and investor confidence and the market price of our ordinary shares may be materially and adversely affected.

If we fail to maintain the adequacy of our internal control over financial reporting, we may not be able to conclude on an ongoing basis that we have effective internal control over financial reporting. If we fail to maintain effective internal control over financial reporting, we could experience material misstatements in our financial statements and fail to meet our reporting obligations, which would likely cause investors to lose confidence in our reported financial information. This could in turn limit our access to capital markets, harm our results of operations, and lead to a decline in the trading price of our ordinary shares. Additionally, ineffective internal control over financial reporting could expose us to increased risk of fraud or misuse of corporate assets and subject us to potential delisting from The Nasdaq Global Select Market, regulatory investigations and civil or criminal sanctions. Our reporting and compliance obligations may place a significant strain on our management, operational and financial resources and systems for the foreseeable future.

Unfavorable global economic conditions, including those caused by political instability in the United States or by the U.K.'s recent departure from the European Union ("Brexit"), could adversely affect our business, financial condition or results of operations.

Our results of operations could be adversely affected by general conditions in the global economy and in the global financial markets. Political instability in the United States and surrounding Brexit has the potential to disrupt global economic conditions and supply changes. While we do not believe that our operations will be directly adversely affected materially by Brexit, we may not be able to anticipate the effects Brexit will have on our suppliers and any collaborators. The most recent global financial crisis caused extreme volatility and disruptions in the capital and credit markets.

A severe or prolonged economic downturn, such as the most recent global financial crisis, could result in a variety of risks to our business, including weakened demand for our product candidates and our ability to raise additional capital when needed on acceptable terms, if at all. A weak or declining economy could strain our suppliers, possibly resulting in supply disruption, or cause delays in payments for our services by third-party payers or our collaborators. Any of the foregoing could harm our business and we cannot anticipate all of the ways in which the current economic climate and financial market conditions could adversely impact our business.

Risks for U.S. Holders

We have in the past qualified and in the future may qualify as a passive foreign investment company, 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 qualified as a passive foreign investment company ("PFIC") for U.S. federal income tax for 2016 but not in 2017, 2018 or 2019. A corporation organized outside the United States generally will be classified as a PFIC for U.S. federal income tax purposes in any taxable year in which at least 75% of its gross income is passive income or on average at least 50% of the gross value of its assets is attributable to assets that produce passive income or are held to produce passive income. Passive income for this purpose generally includes dividends, interest, royalties, rents and gains from commodities and securities transactions. Our status in any taxable year will depend on our assets and activities in each year, and because this is a factual determination made annually after the end of each taxable year, there can be no assurance that we will continue to qualify as a PFIC in future taxable years. The market value of our assets may be determined in large part by reference to the market price of our ordinary shares, which is likely to fluctuate, and may fluctuate considerably given that market prices of biotechnology companies have been especially volatile. If we were considered a PFIC for the current taxable year or any future taxable year, a U.S. holder would be required to file annual information returns for such year, whether the U.S. holder disposed of any ordinary shares or received any distributions in respect of ordinary shares during such year. In certain circumstances a U.S. holder may be able to make certain tax elections that would lessen the adverse impact of PFIC status; however, in order to make such elections the U.S. holder will usually have to have been provided information about the company by us, and we do not intend to provide such information.

The U.S. federal income tax rules relating to PFICs are complex. U.S. holders are urged to consult their tax advisors with respect to the purchase, ownership and disposition of our shares, the possible implications to them of us being treated as a PFIC (including the availability of applicable election, whether making any such election would be advisable in their particular circumstances) as well as the federal, state, local and foreign tax considerations applicable to such holders in connection with the purchase, ownership and disposition of our shares.

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

Although we now report as a U.S. domestic filer for SEC reporting purposes, we are incorporated under the laws of the Netherlands. Some of the members of our board and senior management reside outside the United States. As a result, it may not be possible for shareholders to effect service of process within the United States upon such persons or to enforce judgments against them or us in U.S. courts, including judgments predicated upon the civil liability provisions of the federal securities laws of the United States. In addition, it is not clear whether a Dutch court would impose civil liability on us or any of our Board members in an original action based solely upon the federal securities laws of the United States brought in a court of competent jurisdiction in the Netherlands.

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

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

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

Although we now report as a U.S. domestic filer for SEC purposes, our corporate affairs are governed by our articles of association and by the laws governing companies incorporated in the Netherlands. The rights of our shareholders and the responsibilities of members of our board under Dutch law are different than under the laws of some U.S. jurisdictions. In the performance of their duties, our board members are required by Dutch law to consider the interests of uniQure, its shareholders, its employees and other stakeholders and not only those of our shareholders (as would be required under the law of most U.S. jurisdictions). As a result of these considerations our directors may take action that would be different than those that would be taken by a company organized under the law of some U.S. jurisdictions.

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

None.

Item 3. Defaults Upon Senior Securities

None.

Item 4. Mine Safety Disclosures

Not applicable.

Item 5. Other Information

None.

Item 6. Exhibits

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

EXHIBIT INDEX

101* The following financial information from our Quarterly Report on Form 10-Q for the period ended June 30, 2020, filed with the Securities and Exchange Commission on July 30, 2020, is formatted in Inline Extensible Business Reporting Language ("iXBRL"): (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)

104* The cover page from our Quarterly Report on Form 10-Q for the period ended June 30, 2020, filed with the Securities and Exchange Commission on July 30, 2020, is formatted in Inline Extensible Business Reporting Language ("iXBRL")

† Portions of this exhibit (indicated by asterisks) have been omitted in accordance with the rules of the Securities and Exchange Commission.

* Filed herewith.

± Furnished herewith.

SIGNATURE

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

UNIQURE, N.V.

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

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

Dated July 30, 2020

Portions of this exhibit have been omitted for confidential treatment pursuant to Item 601(b)(10)(iv) of Regulation S-K.

COMMERCIALIZATION AND LICENSE AGREEMENT

BETWEEN

UNIQURE BIOPHARMA BV

AND

CSL BEHRING LLC

Dated June 24, 2020

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COMMERCIALIZATION AND LICENSE AGREEMENT

This COMMERCIALIZATION AND LICENSE AGREEMENT (this "**Agreement**") is made as of June 24, 2020 (the "**Execution Date**") by and between uniQure biopharma BV, a corporation organized under the laws of the Netherlands, having its principal place of business at Paasheuvelweg 25a, 1105 BP Amsterdam, The Netherlands ("**uniQure**"), and CSL Behring LLC, a limited liability company organized and existing under the laws of Delaware, having a registered office located at 1020 First Avenue, King of Prussia, PA 19406, United States ("**Partner**"). uniQure and Partner are referred to in this Agreement individually as a "**Party**" and collectively as the "**Parties**."

RECITALS

WHEREAS, uniQure is a biopharmaceutical company engaged in the Development, Manufacture, and Commercialization of gene therapy products, including a proprietary gene therapy product internally designated as AMT-061;

WHEREAS, uniQure Controls certain Know-How relating to and Patent Rights Covering AMT-061;

WHEREAS, uniQure is conducting the Development of AMT-061 on a global basis to support Regulatory Approvals for any Licensed Product in the Field in the Territory;

WHEREAS, uniQure is seeking a partner to Commercialize any Licensed Product in the Field in the Territory upon the terms and conditions set forth herein;

WHEREAS, Partner is a pharmaceutical company engaged in the Commercialization of biopharmaceutical products in the Territory;

WHEREAS, Partner desires to acquire rights to Commercialize any Licensed Product in the Field in the Territory upon the terms and conditions set forth herein; and

WHEREAS, uniQure desires to grant to Partner, and Partner desires to receive from uniQure, an exclusive right and license under the uniQure Technology to Commercialize any Licensed Product in the Field in the Territory, in each case, upon the terms and conditions set forth herein.

AGREEMENT

NOW, **THEREFORE**, the Parties hereby agree as follows:

ARTICLE 1
DEFINITIONS

Unless specifically set forth to the contrary herein, the following terms will have the respective meanings set forth below, whether used in the singular or plural:

1.1 [*]

1.2 [*]

1.3 "**AAV5 NAb Assay**" means the AAV5 neutralizing antibody assay [*].

1.4 "**Abbreviated Approval Product**" means, with respect to any Licensed Product in a particular country in the Territory, any Gene Therapy Product that (a) is marketed for the prevention, treatment or cure of Hemophilia B and sold by a Third Party that is not a Sublicensee of, or a Third Party Distributor for, Partner or any of its Affiliates (and such Gene Therapy Product is not otherwise licensed, supplied, or otherwise permitted or marketed by a Party or its Affiliates or Sublicensees), (b) comprises an AAV5 viral vector carrying the Padua variant of the Factor IX gene, and (c) is approved through an abbreviated process (such as, in the United States, a "Biosimilar Biologic Product" under the Patient Protection and Affordable Care Act, as amended by the Health Care and Education Reconciliation Act of 2010, Title VII, Subtitle A, Biologics Price Competition and Innovation Act of 2009 (codified, in part at 42 U.S.C. § 262), or, outside the United States, in accordance with European Directive 2001/83/EC on the European Community Code for medicinal products (Article 10(4) and Section 4, Part II of Annex I) and European Regulation EEC/2309/93 establishing the European Community procedures for the authorization and evaluation of medicinal products, each as amended, and together with all associated final guidance documents, and any counterparts thereof or equivalent process to the foregoing inside or outside of the United States or E.U.).

1.5 "**Accounting Standards**" means GAAP or IFRS (as applicable to a Party).

1.6 "**Affiliates**" of a Person from time to time means any other Person that (directly or indirectly) is controlled by, controls, or is under common control with such Person at such time. For the purposes of this definition only, the term "control" (including, with correlative meanings, the terms "controlled by" and "under common control with") as used with respect to a Person, means the possession, directly or indirectly, of the power to direct, or cause the direction of, the management or policies of such Person, whether through the ownership of voting securities, by contract or otherwise, and "control" will be presumed to exist if either of the following conditions is met: (a) in the case of a corporate entity, direct or indirect ownership of voting securities entitled to cast more than [*] percent ([*]%) of the votes in the election of directors or (b) in the case of a non-corporate entity, direct or indirect ownership of more than [*] percent ([*]%) of the equity interests with the power to direct the management and policies of such entity. For all purposes of this Agreement, uniQure or its Affiliates will not be an Affiliate of Partner or any of Partner's Affiliates, and Partner or its Affiliates will not be an Affiliate of uniQure or any of uniQure's Affiliates.

1.7 "**Agreement**" has the meaning set forth in the Preamble.

1.8 "**Alleged Party**" has the meaning set forth in Section 13.2.2(b) (Termination for Cause).

1.9 "**Alleging Party**" has the meaning set forth in Section 13.2.2(b) (Termination for Cause).

1.10 "**Alliance Manager**" has the meaning set forth in Section 7.1 (Alliance Managers).

1.11 "**Allowable Overruns**" means, with respect to the uniQure Development Budget (including the Current Phase III Budget or any uniQure Additional Development Budget, as applicable), any actual internal costs (at the FTE Rate) or documented out-of-pocket Third Party expenses incurred by or on behalf of uniQure in the performance of any uniQure Development Activities in accordance with the Current Phase III Protocol or the uniQure Additional Development Plan (as applicable) that is within [*]% of the aggregate amount budgeted in the Current Phase III Budget or uniQure Additional Development Budget (as applicable).

1.12 "**Alternative Transaction**" means (a) any sale, assignment, transfer, or other disposition (howsoever effected), including any exclusive or co-exclusive license, of all or a material portion of the Commercialization rights within the United States to AMT-061 by uniQure or any of its Affiliates to a Third Party, (b) any sale, assignment, transfer, or other disposition (howsoever effected) to a Third Party of (i) control (within the meaning set forth in the definition of Affiliate) of uniQure, or (ii) all or substantially all of the assets of uniQure.

1.13 "**AMT-061**" means uniQure's proprietary gene therapy product known as etranacogene dezaparvovec, consisting of an AAV5 viral vector carrying a gene cassette with the LP-1 promoter and the Padua variant of Factor IX (FIX-Padua).

1.14 "**Anti-Corruption Laws**" means any local and other anti-corruption laws, including the provisions of the United States Foreign Corrupt Practices Act, as amended.

1.15 "**Antitrust Agencies**" means the Competition and Markets Authority of the United Kingdom, the United States Federal Trade Commission, the United States Department of Justice, and the Australian Competition and Consumer Commission.

1.16 "**Antitrust Clearance Date**" means the earliest date on which all applicable waiting periods and approvals (including confirmations that an Antitrust Agency does not oppose the transactions contemplated by this Agreement) required or advisable under Antitrust Laws in the U.S., the United Kingdom, and Australia with respect to the transactions contemplated under this Agreement have expired, been terminated (in the case of waiting periods) or been received (in the case of approvals), or been terminated because the transactions contemplated by this Agreement are found not to qualify for review.

1.17 "**Antitrust Filing**" means filings by uniQure and Partner with the Competition and Markets Authority of the United Kingdom, the United States Federal Trade Commission, the United States Department of Justice, the Australian Competition and Consumer Commission, as required or advisable under any Antitrust Laws with respect to the transactions contemplated under this Agreement, together with all required documentary attachments thereto.

1.18 "**Antitrust Laws**" means any and all Applicable Laws designed to prohibit, restrict, or regulate actions for the purpose or effect of monopolization, merger control, or restraint of trade.

1.19 "**Applicable Law**" means collectively all laws, statutes, rules, regulations, ordinances, decrees, and judicial and administrative orders and judgments of any applicable Governmental Authority that govern or otherwise apply to a Party or the activities contemplated herein, including all Antitrust Laws and Anti-Corruption Laws.

1.20 "**Assigned Regulatory Materials**" has the meaning set forth in Section 3.5.1 (Regulatory Transfer).

1.21 "**Business Day**" means a day other than a Saturday, Sunday, or a day on which banking institutions in New York, New York (USA) or Amsterdam, Netherlands are authorized or required by Applicable Law to remain closed.

1.22 "**Buyers**" has the meaning set forth in Section 1.117 (Net Sales).

1.23 "**cGMP**" means all applicable then-current laws and guidelines applicable to the Manufacture of the Licensed Product, including, as applicable, (a) the principles detailed in the U.S. Current Good Manufacturing Practices, 21 C.F.R. Parts 4, 210, 211, 601, 606, 610 and 820, (b) European Directive 2003/94/EC and Eudralex 4, (c) the principles detailed in the International Conference on Harmonization's Q7 guidelines, (d) the FD&C Act and those standards required by the FDA, and (e) the equivalent Applicable Law in any relevant country or region, each as may be amended and applicable from time to time.

1.24 "**Change of Control**" means, with respect to a Party, that: (a) any Third Party acquires directly or indirectly the beneficial ownership of any voting security of such Party, or, if the percentage ownership of such Third Party in the voting securities of such Party is increased through stock repurchase, redemption, cancellation, recapitalization, reorganization, or other action affecting the capital stock of such Party and immediately thereafter such Third Party is, directly or indirectly, the beneficial owner of voting securities representing more than 50% of the total voting power of all of the then outstanding voting securities of such Party; (b) a merger, consolidation, recapitalization, reorganization or other business combination of or involving such Party is consummated that results in shareholders or equity holders of such Party immediately prior to such transaction, ceasing to own more than 50% of the combined outstanding voting securities of the surviving entity (or its parent entity) immediately following such transaction; or (c) there is a sale or transfer to a Third Party of all or substantially all of such Party's consolidated assets taken as a whole, through one or more related transactions; for the purposes hereof, the term "voting security" of a Party means any security that entitles the holder thereof to vote on the election of directors of such Party.

1.25 "**Clinical Trial**" means any clinical trial in humans that is conducted in accordance with GCP and is designed to generate data in support or maintenance of an IND, MAA, or other similar marketing application or Regulatory Approval, whether prior to or after receipt of Regulatory Approval for a pharmaceutical or biologic product.

1.26 "**CMO**" means a contract manufacturing organization.

1.27 "**Commercialization**" means any and all activities directed to the marketing, promotion, distribution, pricing, importing, exporting, reimbursement, offering for sale, and sale of a

pharmaceutical or biologic product and interacting with Regulatory Authorities following receipt of Regulatory Approval in the applicable country or region for such pharmaceutical or biologic product regarding the foregoing, including seeking any required Reimbursement Approval and all post-marketing surveillance, but excluding activities that constitute Manufacturing, Development, or performance of Medical Affairs. "Commercialize," "Commercializing," and "Commercialized" will be construed accordingly.

1.28 "**Commercially Reasonable Efforts**" means, with respect to the Exploitation of a Licensed Product by a Party, those efforts and resources, including allocation of reasonably necessary personnel, equivalent to the efforts that a reasonable global biopharmaceutical company or a pharmaceutical company, in each case, that is of comparable size and resources to such Party would typically devote as part of an active and continuing program of development and commercialization of a pharmaceutical or biologic product of similar market potential, at a similar stage of its product life and taking into account all relevant factors, facts, and circumstances, including the competitiveness of the marketplace and the proprietary position, product profile, the patent or other intellectual property status (including the strength and duration of patent protection and anticipated exclusivity), the likelihood and timing of Regulatory Approvals and Reimbursement Approvals, the current guidance and requirements for Regulatory Approvals and Reimbursement Approvals, the then and projected regulatory status, the ability to Manufacture or have Manufactured such product, labeling considerations, safety, tolerability, stability and efficacy, present and future market potential and performance, existing and projected pricing, sales, reimbursement and profitability, pricing or reimbursement changes in relevant countries or regulatory jurisdictions. Commercially Reasonable Efforts requires, with respect to an obligation, that the Party: (a) promptly assign responsibility for such obligation to specific employees who are held accountable for progress and monitor such progress on an on-going basis, (b) set and consistently seek to achieve specific and meaningful objectives for carrying out such obligation, and (c) consistently make and implement decisions and allocate resources designed to advance progress with respect to such objectives; in each case ((a), (b), and (c)), consistent with such Party's usual business practice. Notwithstanding the forgoing or any provision in this Agreement to the contrary, (i) Partner shall not be in breach of its obligations to use Commercially Reasonable Efforts in respect of any Development, Manufacturing, Commercialization or any other activities to the extent a delay in Partner's performance is caused by a failure or delay in the performance by uniQure of any of its obligations under this Agreement, and (ii) in a determination of an expenditure of Commercially Reasonable Efforts, a Party may not take into account any of its own other products (including any other product for Hemophilia B).

1.29 "**Committees**" has the meaning set forth in Section 7.7 (Committees).

1.30 "**Competitive Product**" means any [*] for the treatment, prevention, or cure of Hemophilia B.

1.31 "**Complete Regulatory Files**" has the meaning set forth in Section 3.5.1 (Regulatory Transfer).

1.32 "**Confidential Disclosure Agreement**" means that certain Confidential Disclosure Agreement by and between uniQure and Partner effective on November 11, 2019.

1.33 "**Confidential Information**" means, subject to Section 9.3 (Exemptions), (a) Know-How and any technical, scientific, trade, research, manufacturing, business, financial, marketing, product, supplier, intellectual property, and other non-public or proprietary data or information (including unpublished patent applications) that may be disclosed by or on behalf of one Party or its Affiliates to the other Party or its Affiliates pursuant to this Agreement or the transactions contemplated hereby (including information disclosed prior to the Execution Date pursuant to the Confidential Disclosure Agreement), regardless of whether such information is specifically marked or designated as confidential and regardless of whether such information is in written, oral, electronic, or other form, and (b) the terms of this Agreement.

1.34 "**Control**" or "**Controlled**" means (a) the possession by a Party or its Affiliates (whether by ownership, license, or otherwise other than pursuant to this Agreement) of, (i) with respect to any tangible Know-How, the legal authority or right to physical possession of such tangible Know-How, with the right to provide such tangible Know-How to the other Party on the terms set forth herein, or (ii) with respect to Patent Rights, Regulatory Approvals, Reimbursement Approvals, Regulatory Submissions, intangible Know-How, or other intellectual property rights, the legal authority or right to grant a license, sublicense, access, right of reference, or right to use (as applicable) to the other Party under such Patent Rights, Regulatory Approvals, Regulatory Submissions, intangible Know-How, or other intellectual property rights on the terms set forth herein, in each case ((i) and (ii)), without breaching or otherwise violating the terms of any arrangement or agreement with a Third Party in existence as of the time such Party or its or Affiliates would first be required hereunder to grant the other Party such access, right of reference, right to use, licenses, or sublicense and without being required to make any additional payment to any Third Party and (b) with respect to any product, the possession by a Party or its Affiliates of the ability (whether by sole or joint ownership, license, or otherwise, other than pursuant to the licenses granted under this Agreement) to grant an exclusive (or non-exclusive, as applicable) license or sublicense of Patent Rights that Cover such product or proprietary Know-How that is used in connection with the Exploitation of such product. Notwithstanding the foregoing, a Party and its Affiliates will not be deemed to "Control" any Patent Right, Know-How, or product that, prior to the consummation of a Change of Control of such Party, is owned or in-licensed by a Third Party that becomes an Affiliate of such acquired Party after the Effective Date as a result of such Change of Control unless (A) prior to the consummation of such Change of Control, such acquired Party or any of its Affiliates also Controlled such Patent Right, Know-How, or product, or (B) the Know-How, Patent Rights, or product owned or in-licensed by the applicable Third Party were not used by the acquired Party or any of its Affiliates (as defined prior to the consummation of such Change of Control) in the performance of activities under this Agreement prior to the consummation of such Change of Control, but after the consummation of such Change of Control, such acquired Party or any of its Affiliates determines to use or uses any such Patent Rights, Know-How, or product in the performance of its obligations or exercise of its rights under this Agreement, in each of which cases ((A) and (B)), such Patent Rights, Know-How, or product will be "Controlled" by such Party for purposes of this Agreement.

1.35 "**Controlling Party**" has the meaning set forth in Section 12.5.1 (Notice).

1.36 "**Cover**" means (a) with respect to a particular subject matter at issue and a relevant Patent Right, that the manufacture, use, sale, offer for sale, or importation of such subject matter would fall within the scope of one or more claims in such Patent Right.

1.37 "**CRE Cure Plan**" has the meaning set forth in Section 13.2.2(b) (Termination for Cause).

1.38 "**CRE Default**" has the meaning set forth in Section 13.2.2(b) (Termination for Cause).

1.39 "**CRE Default Notification**" has the meaning set forth in Section 13.2.2(b) (Termination for Cause).

1.40 "**CRE Explanation**" has the meaning set forth in Section 13.2.2(b) (Termination for Cause).

1.41 "**CREATE Act**" has the meaning set forth in Section 12.3 (CREATE Act).

1.42 "**Current Phase III Budget**" means the budget of all internal costs (at the FTE Rate) and Third Party expenses to be incurred after the Effective Date in the performance of the Current Phase III Protocol, as such budget may be updated in accordance with this Agreement.

1.43 "**Current Phase III Protocol**" means the protocol as of the Effective Date or as may be amended during the Term in accordance with this Agreement for the Phase III Clinical Trial for which uniQure is the sponsor for the Lead Product in the Field registered at www.clinicaltrials.gov as "HOPE-B: Trial of AMT-061 in Severe or Moderately Severe Hemophilia B Patients."

1.44 "**Debarred/Excluded**" means any Person (a) becoming debarred or suspended under 21 U.S.C. §§335a(a) or (b), the subject of a conviction described in 21 U.S.C. §335a, disqualified by FDA, excluded, or having previously been excluded, from a U.S. federal or governmental health care program, debarred from U.S. federal contracting, convicted of or pled *nolo contendere* to any felony, or to any U.S. federal or state legal violation (including misdemeanors) relating to medical drug products or services or fraud, subject to OFAC sanctions or on the OFAC list of specially designated nationals, or (b) the subject of any similar sanction of any Governmental Authority in the Territory.

1.45 "**Default**" has the meaning set forth in Section 13.2.2(a) (Termination for Cause).

1.46 "**Default Notification**" has the meaning set forth in Section 13.2.2(a) (Termination for Cause).

1.47 "**Development**" means all internal and external research, development, and regulatory activities related to pharmaceutical or biologic products, including (a) non-clinical testing, toxicology, testing and studies, non-clinical and preclinical activities, and Clinical Trials, and (b) preparation, submission, review, and development of data or information for the purpose of submission to a Regulatory Authority to obtain authorization to conduct Clinical

Trials or to obtain, support, or maintain Regulatory Approval of a pharmaceutical or biologic product. Development will include development and regulatory activities for additional forms, formulations, or indications for a pharmaceutical or biologic product after receipt of Regulatory Approval of such product (including label expansion), including Clinical Trials initiated following the receipt of Regulatory Approval or any Clinical Trial to be conducted after receipt of Regulatory Approval that was mandated by the applicable Regulatory Authority as a condition of receipt of such Regulatory Approval with respect to an approved formulation or indication. Development excludes activities that constitute Manufacturing, performance of Medical Affairs, or Commercialization. "Develop," "Developing," and "Developed" will be construed accordingly.

1.48 "**Development Payment**" has the meaning set forth in Section 8.4 (Development Payment).

1.49 "**Disclosing Party**" has the meaning set forth in Section 9.1.1 (Duty of Confidence).

1.50 "**Dispute**" has the meaning set forth in Section 14.1 (Dispute Resolution; General).

1.51 "**Dollar**" means the U.S. dollar, and "$" will be interpreted accordingly.

1.52 "**Effective Date**" has the meaning set forth in Section 15.1 (Effective Date).

1.53 "**EMA**" means the European Medicines Agency or any successor agency thereto.

1.54 "**Encumbrance**" means a lien, charge, security interest, mortgage, pledge, liability, or other encumbrance or right exercisable by a Third Party having similar effect.

1.55 "**Escalation Index**" means with respect to uniQure, the Consumer Price Index-Urban Wage Earners and Clerical Workers, U.S. City Average, All Items 1982-84=100, unadjusted indexes, published by the United States Department of Labor, Bureau of Labor Statistics (or its successor equivalent index).

1.56 "**European Union**" or "**E.U.**" means the economic, scientific, and political organization of member states of the European Union as it may be constituted from time to time.

1.57 "**Examined Party**" has the meaning set forth in Section 8.12 (Financial Records and Audits).

1.58 "**Execution Date**" has the meaning set forth in the Preamble.

1.59 "**Executive Officer**" means (a) in the case of uniQure, the chief executive officer of uniQure N.V and (b) in the case of Partner, the chief executive officer of CSL Limited, *provided* that each of them may delegate any matter to an appropriate executive officer or other senior member of management, in which case such delegee will be deemed the Executive Officer for such matter.

1.60 "**Existing In-Licenses**" means any agreement entered into by uniQure or its Affiliates with a Third Party prior to the Execution Date, including any amendments or restatements

thereto entered into during the Term, pursuant to which uniQure or its Affiliate Controls any uniQure Technology.

1.61 "**Existing Product**" has the meaning set forth in Section 13.3.1 (Licenses).

1.62 "**Exploit**" means to make, have made, use, offer to sell, sell, Develop, Manufacture, perform Medical Affairs, Commercialize, or otherwise exploit. "Exploitation" will be construed accordingly.

1.63 "**Factor IX**" means the serine protease of the coagulation system commonly referred to as "factor IX".

1.64 "**FD&C Act**" means the United States Federal Food, Drug and Cosmetic Act, as amended from time to time, together with any rules, regulations, and requirements promulgated thereunder (including all additions, supplements, extensions, and modifications thereto).

1.65 "**FDA**" means the United States Food and Drug Administration or any successor entity thereto having essentially the same function.

1.66 "**Field**" means the diagnosis, treatment, prevention, or cure of any human diseases or medical conditions.

1.67 "**Financial Quarter**" means the respective periods of three consecutive calendar months ending on September 30, December 31, March 31 and June 30.

1.68 "**Financial Year**" means each 12-month period commencing on July 1.

1.69 "**First Commercial Sale**" means, with respect to a Licensed Product or an Abbreviated Approval Product in any country, the first sale of such Licensed Product or Abbreviated Approval Product (as applicable) to a Third Party for distribution or use in such country after receipt of Regulatory Approvals (and, if applicable in such country, Reimbursement Approvals) for such Licensed Product or Abbreviated Approval Product (as applicable) in such country. First Commercial Sale excludes any sale or other distribution of a Licensed Product or Abbreviated Approval Product for use in a Clinical Trial or other Development activity.

1.70 "**First Major Regulatory Approval**" means the First Regulatory Approval of a Licensed Product to be obtained in any of the Major Countries and, if applicable in such Major Country, the first Reimbursement Approval of such Licensed Product in such Major Country.

1.71 "**Flash Report**" has the meaning set forth in Section 8.3.3(a) (Flash Reports).

1.72 "**Force Majeure**" has the meaning set forth in Section 16.7 (Force Majeure).

1.73 "**FTC**" has the meaning set forth in Section 15.2 (Antitrust Filings).

1.74 "**FTE**" means the equivalent of the work of one duly qualified employee of a Party full time for one year (consisting of a total of 1,800 hours per year) carrying out Development or Manufacturing or other scientific or technical work under this Agreement. Overtime and work on weekends, holidays, and the like, in each case, will not be counted with any multiplier (*e.g.*, time-and-a-half or double time) toward the number of hours that are used to calculate the FTE contribution. The portion of an FTE billable by a Party for one individual during a given accounting period will be determined by dividing the number of hours worked directly by such individual on the work to be conducted under this Agreement during such accounting period and the number of FTE hours applicable for such accounting period based on 1,800 working hours per Financial Year.

1.75 "**FTE Rate**" means the amount for an FTE per Financial Year, which for the Financial Year ending on June 30, 2021 will be [*] per FTE, pro-rated for the period beginning on the Effective Date and ending on June 30, 2021. Beginning on July 1, 2021 and on July 1 of each subsequent Financial Year during the Term, each FTE Rate is subject to annual adjustment by the percentage increase or decrease in the Escalation Index comparing the levels of the Escalation Index as of June 30 of the two most recently completed Financial Years.

1.76 "**GAAP**" means the generally accepted accounting principles in the United States, consistently applied.

1.77 "**GCP**" means all applicable good clinical practice standards for the design, conduct, performance, monitoring, auditing, recording, analyses and reporting of Clinical Trials, including, as applicable (a) as set forth in the International Conference on Harmonization of Technical Requirements for Registration of Pharmaceuticals for Human Use Harmonized Tripartite Guideline for Good Clinical Practice (CPMP/ICH/135/95) (the "**ICH Guidelines**") and any other guidelines for good clinical practice for trials on medicinal products in the Territory, (b) the Declaration of Helsinki (2013) as last amended at the 52nd World Medical Association in October 2000 and any further amendments or clarifications thereto, (c) U.S. Code of Federal Regulations Title 21, Parts 11 (Electronic Records; Electronic Signatures), 50 (Protection of Human Subjects), 54 (Financial Disclosure by Clinical Investigators), 56 (Institutional Review Boards), 312 (Investigational New Drug Application), 601 (Applications for FDA Approval of a Biologic License), as may be amended from time to time, and (d) the equivalent Applicable Law in the Territory, each as may be amended and applicable from time to time and in each case, that provide for, among other things, assurance that the clinical data and reported results are credible and accurate and have data integrity and protect the rights, integrity, and confidentiality of trial subjects.

1.78 [*]

1.79 "**Gene Therapy Product**" means [*].

1.80 "**GLP**" means all applicable good laboratory practice standards, including, as applicable, as set forth in the then-current good laboratory practice standards promulgated or endorsed by the U.S. Food and Drug Administration, as defined in 21 C.F.R. Part 58, and the

equivalent Applicable Law in the region in the Territory, each as may be amended and applicable from time to time.

1.81 "**Governmental Authority**" means any federal, national, state, provincial, or local government, or political subdivision thereof, or any multinational organization or any authority, agency, regulatory body, or commission entitled to exercise any administrative, executive, judicial, legislative, police, regulatory or taxing authority or power, or any court or tribunal (or any department, bureau or division of any of the foregoing, or any governmental arbitrator or arbitral body). Governmental Authorities include all Regulatory Authorities.

1.82 "**Hemophilia B**" means the genetic bleeding disorder caused by missing or defective blood clotting Factor IX known as hemophilia B.

1.83 "**ICC**" has the meaning set forth in Section 14.2 (Arbitration).

1.84 "**ICC Court**" means the International Court of Arbitration of the ICC.

1.85 "**IFRS**" means International Financial Reporting Standards as endorsed by the EU, consistently applied.

1.86 "**Incremental Withholding Amount**" has the meaning set forth in Section 8.13 (Taxes).

1.87 "**IND**" means an Investigational New Drug application required pursuant to 21 C.F.R. Part 312 or any comparable filings outside of the U.S. (such as an application for a Clinical Trial authorization in the E.U.).

1.88 "**Indemnified Party**" has the meaning set forth in Section 11.3 (Indemnification Procedure).

1.89 "**Indemnifying Party**" has the meaning set forth in Section 11.3 (Indemnification Procedure).

1.90 "**Initial E.U. MAA**" has the meaning set forth in Section 3.2.1 (Preparation of the Initial U.S. MAA and the Initial E.U. MAA).

1.91 "**Initial U.S. MAA**" has the meaning set forth in Section 3.2.1 (Preparation of the Initial U.S. MAA and the Initial E.U. MAA).

1.92 "**Invention**" means any new and useful process, method, manufacture, or composition of matter, know-how, or other invention that is conceived and first reduced to practice, constructively or actually, by either Party or jointly by the Parties in connection with the performance of activities under this Agreement.

1.93 "**Joint Know-How**" means any Know-How, developed or invented during the Term in the performance of activities under this Agreement jointly by a Party or such Party's or its Affiliates', licensees', Sublicensees', or Subcontractors' employees, agents, or independent contractors, or any other Persons, in each case, that are contractually required

to assign such Know-How to such Party or any Affiliate of such Party, on the one hand, and the other Party or such Party's or its Affiliates', licensees', Sublicensees', or Subcontractors' employees, agents, or independent contractors, or other Persons, in each case, that are contractually required to assign such Know-How to such Party or any Affiliate of such Party, on the other hand.

1.94 "**Joint Patent Rights**" means any Patent Right that has a priority date after the Effective Date, and that Covers any Invention included in the Joint Know-How.

1.95 "**Joint Technology**" means the Joint Know-How and the Joint Patent Rights.

1.96 "**JSC**" has the meaning set forth in Section 7.2.1 (Formation and Purpose of JSC).

1.97 "**Know-How**" means any proprietary information or materials, including records, discoveries, improvements, modifications, processes, techniques, methods, assays, chemical or biological materials, designs, protocols, formulas, data (including physical data, chemical data, toxicology data, animal data, raw data, clinical data, and analytical and quality control data), dosage regimens, control assays, product specifications, marketing, pricing and distribution costs, Inventions, algorithms, technology, forecasts, profiles, strategies, plans, results in any form whatsoever, know-how and trade secrets (in each case, patentable, copyrightable or otherwise).

1.98 "**Knowledge**" means (a) with respect to uniQure, the actual knowledge, as of the Execution Date, of the uniQure individuals in the roles set forth in part (a)(i) of Schedule 1.98, and, for the purposes of Section 10.2.6 (Representations and Warranties of uniQure), the individuals in the roles set forth in part (a)(ii) of Schedule 1.98, and (b) with respect to Partner, the actual knowledge, as of the Execution Date, of the Partner individuals in the roles set forth in part (b) of Schedule 1.98 as of such date.

1.99 "**Lead Product**" means AMT-061, in any dosage strength, concentration, or formulation.

1.100 "**Licensed Product**" means (a) the Lead Product and (b) any Variant.

1.101 "**Loss of Market Exclusivity**" means, with respect to a Licensed Product in a country in the Territory, the date on which (a) one or more Abbreviated Approval Products are being marketed in such country; and (b) the number of unit equivalents of such Abbreviated Approval Products sold in such country in a particular Financial Quarter equals or exceeds [*]% of the number of units of such Licensed Product sold in such country in such Financial Quarter.

1.102 "**Losses**" means damages, debts, obligations, and other liabilities, losses, claims, taxes, interest obligations, deficiencies, judgments, assessments, fines, fees, penalties, or expenses (including amounts paid in settlement, interest, court costs, costs of investigators, reasonable fees and expenses of attorneys, accountants, financial advisors, consultants, and other experts, and other expenses of litigation), subject to Section 16.2 (Limitation of Liability).

1.103 "**Major Country**" means each of [*].

1.104 "**Manufacture**" means activities directed to manufacturing, processing, packaging, labeling, filling, finishing, assembly, shipping, storage, or freight of any pharmaceutical or biologic product (or any components or process steps involving any such product), placebo, or comparator agent, as the case may be, including quality assurance and stability testing, characterization testing, quality control release testing of drug or biologic substance and drug or biologic product, quality assurance batch record review and release of product, process development, qualification, and validation, scale-up, pre-clinical, clinical, and commercial manufacture and analytic development, and product characterization, but excluding activities that constitute Development, performance of Medical Affairs, or Commercialization. "Manufacturing" and "Manufactured" will be construed accordingly.

1.105 "**Manufacturing Development Plan**" has the meaning set forth in Section 5.2 (Manufacturing Development Plan).

1.106 "**Manufacturing Development Plan Required Change Amount**" has the meaning set forth in Section 7.5.2(b)(ii) (Partner Decisions).

1.107 "**Manufacturing Process**" has the meaning set forth in the Supply Agreement.

1.108 "**Manufacturing Responsibility Cutover Date**" has the meaning set forth in Section 5.3.3 (Manufacturing Responsibility Transfer Plan).

1.109 "**Manufacturing Responsibility Transfer Notice Date**" has the meaning set forth in Section 5.3.2 (Transfer of Manufacturing Responsibility).

1.110 "**Manufacturing Responsibility Transfer Plan**" has the meaning set forth in Section 5.3.3 (Manufacturing Responsibility Transfer Plan).

1.111 "**Mark**" means any trademark, trade name, service mark, service name, product name, brand, domain name, trade dress, logo, slogan, or other indicia of origin or ownership, and (a) all registrations, applications for registrations, and other similar intellectual property rights associated with any of the foregoing, and (b) the goodwill associated with each of the foregoing.

1.112 "**Marketing Authorization Application**" or "**MAA**" means any biologics license application or other marketing authorization application, in each case, filed with the applicable Regulatory Authority in a country or other regulatory jurisdiction, which application is required to commercially market or sell a pharmaceutical or biologic product in such country or jurisdiction (and any amendments thereto), including (i) any marketing authorization application filed with the EMA under the centralized EMA filing procedure to gain approval to market a pharmaceutical or biologic product in the E.U. or a Regulatory Authority in any E.U. country if the centralized EMA filing procedure is not used to gain approval to market a pharmaceutical or biologic product in the E.U. and (ii) all Biologics License Applications ("**BLAs**") or equivalent submitted to the FDA in the United States in accordance with 42 U.S.C. § 262 and, in each case, any amendments thereto and supplemental applications, but excluding Reimbursement Approval applications.

1.113 "**Material Change to the Manufacturing Section**" has the meaning set forth in Section 3.3 (Other Regulatory Submissions).

1.114 "**Medical Affairs**" means activities conducted by a Party's medical affairs departments (or, if a Party does not have a medical affairs department, the equivalent function thereof), including communications with key opinion leaders, medical education, symposia, advisory boards (to the extent related to medical affairs or clinical guidance), activities performed in connection with patient registries, and other medical programs and communications, including educational grants, research grants (including conducting investigator-initiated studies), and charitable donations to the extent targeted or directed to medical affairs.

1.115 "**Milestone Events**" has the meaning set forth in Section 8.2.3 (Notification of Milestone Events).

1.116 "**Milestone Payment**" has the meaning set forth in Section 8.2.3 (Notification of Milestone Events).

1.117 "**Net Sales**" means with respect to a Licensed Product, the gross amount invoiced or received by or for the benefit of Partner and its Affiliates and Sublicensees (each of the foregoing, a "**Seller**") to a Third Party (including Third Party Distributors) ("**Buyers**") in *bona fide* arm's length transactions within the Territory with respect to such Licensed Product, less the following deductions, in each case, to the extent actually allowed and taken by such Buyers and not otherwise recovered by or reimbursed to Seller in connection with such Licensed Product:

(a) transportation, shipping, freight, handling, and insurance costs incurred in transporting such Licensed Product to Buyers, to the extent actually incurred and itemized;

(b) sales, excise taxes, tariffs, and duties paid by the Seller and any other governmental charges or taxes imposed specifically upon the sale, transportation, delivery, use, exportation, or importation of such Licensed Product and actually paid;

(c) usual and customary discounts and rebates actually allowed and taken (including trade, cash and quantity discounts and rebates) in connection with the sale of such Licensed Product to the extent not attributable to other products of Partner or its Affiliates;

(d) sales returns, allowances, refunds, or credits to such Buyer actually given or other amounts actually repaid by Seller and not in excess of the selling price of such Licensed Product on account of inefficacy (in whole or in part) rejection, outdating, recalls, price adjustments, or billing errors of or with respect to such Licensed Product;

(e) discounts actually paid under government-legislated or Seller-sponsored discount prescription drug programs or other similar coupon or voucher programs;

(f) rebates, reimbursements, fees, clawbacks, discounts, charge-backs, or similar payments paid or credited to Third Party Distributors, pharmacies and other retailers, buying groups (including group purchasing organizations), health care insurance carriers, Third Party payor, administrator, or contractee, pharmacy benefit management companies, health maintenance organizations, Governmental Authorities, hospitals, or other institutions or health care organizations, to the extent such payments are not attributable to other products of Partner or its Affiliates or Sublicensees or other services of Partner or its Affiliates or Sublicensees that are unrelated to the sales and distribution of the Licensed Product;

(g) bad debt expenses and amounts actually written off as uncollectible; and

(h) expenses actually incurred by a Seller [*], up to the lesser of (i) [*] or (ii) [*] less any deductions pursuant to clauses (a) through (g) above.

If Seller receives non-cash consideration for a Licensed Product sold to a Buyer during the Term, then the Net Sales amount for such Licensed Product will be calculated based on the average arms-length cash selling price for such Licensed Product over the immediately prior four Financial Quarters in the relevant countries.

No deduction will be made for any item of cost incurred by any Seller in Developing or Commercializing Licensed Product except as permitted pursuant to clauses (a) through (h) above; *provided* that (x) Licensed Product transferred to Buyers in reasonable quantities in connection with Clinical Trials or other Development activities and (y) any compassionate sales or uses and indigent patient programs, in each case, will not give rise to Net Sales except to the extent that [*]. If a single item falls into more than one of the categories set forth in clauses (a) through (h) above, then such item may not be deducted more than once.

If a Licensed Product is sold in combination with one or more other products of Partner or its Affiliates or Sublicensees, then the gross amount invoiced or received and any deductions in clauses (a) through (h) above applicable to such combined sale will be fairly and equitably allocated by Partner in good faith between such Licensed Product and other products of Partner and its Affiliates and Sublicensees that were included in such combined sale such that such Licensed Product does not bear a disproportionate portion of such deductions.

Calculations of Net Sales will be consistently applied across all products of Seller and will be consistent between periods.

Such amounts will be determined from the books and records of the applicable Seller and will be calculated in accordance with applicable Accounting Standards.

Transfers or sales between Partner and its Affiliates and Sublicensees will be disregarded for purposes of calculating Net Sales, except if such purchaser is an end user (subject to the other limitations above).

If any deductions in (x) clauses (a) through (e) above relating to any Licensed Product sold to a Buyer during the Term is incurred or otherwise becomes due by Seller within [*]

following the end of the Term or (y) clauses (f) or (h) above relating to any Licensed Product sold to a Buyer during the Term is incurred or otherwise becomes due by Seller within [*] following the end of the Term, then, in each case ((x) and (y)), such deduction shall be deemed to have been incurred or to have become due during the Term and such amount may be deducted from any payment to uniQure hereunder (or, if there is no such payment payable, may be invoiced by Partner to uniQure pursuant to Section 8.5 (Other Amounts Payable)). Upon uniQure's request at any time during the Term and for [*] thereafter (such requests to be no more frequent than once per Financial Year), Partner will provide a good faith non-binding estimate of any such anticipated deductions for use in preparing uniQure's financial statements.

1.118 "**OFAC**" means the Office of Foreign Assets Control of the United States Department of the Treasury or any successor agency thereto.

1.119 "**Outside Date**" has the meaning set forth in Section 15.5 (Outside Date).

1.120 "**Partner**" has the meaning set forth in the Preamble.

1.121 "**Partner Commercialization Plan**" has the meaning set forth in Section 6.4 (Commercialization Plan).

1.122 "**Partner Development Plan**" has the meaning set forth in Section 4.2 (Partner Development Plan).

1.123 "**Partner Housemarks**" means (a) the corporate logo of Partner or any of its Affiliates, (b) the trademarks "CSL" and "CSL BEHRING", (c) any other Mark (whether registered or unregistered) containing the word "CSL" or "BEHRING", (d) any other corporate logo or any other Mark (i) used by Partner or any of its Affiliates to identify Partner or its Affiliates or to refer to any product (other than a Licensed Product) or (ii) used by Partner or any of its Affiliates with patient support or other information or services or Promotional Materials associated with any product (other than a Licensed Product), (e) all registrations, applications for registrations, and other similar intellectual property rights associated with any of the foregoing, and (f) all goodwill associated with any and all of the foregoing in clauses (a) through (e).

1.124 "**Partner Indemnitee(s)**" has the meaning set forth in Section 11.2 (Indemnification; By uniQure).

1.125 "**Partner Know-How**" means all Know-How (excluding Partner's interest in any Joint Know-How) that (i) is Controlled by Partner or any of its Affiliates as of the Effective Date or during the Term, and (ii) is necessary or reasonably useful for the Exploitation of any Licensed Product.

1.126 "**Partner Manufacturing Improvements**" means any Invention that is an improvement to the method used in Manufacturing any Licensed Product (or any components thereof); that is developed or invented during the Term by Partner's or its Affiliates', licensees', Sublicensees', or Subcontractors' employees, agents, or independent contractors, or any other Person, in each case, if such Person is contractually required to assign or license

such Invention to Partner or any Affiliate of Partner, whether solely or jointly with others, in the course of performance of any activities under this Agreement.

1.127 "**Partner Manufacturing Patent Right**" means any Patent Right with a priority date after the Effective Date that Covers any Partner Manufacturing Improvement.

1.128 "**Partner Manufacturing Technology**" means Partner Manufacturing Improvements and Partner Manufacturing Patent Rights.

1.129 "**Partner Patent Rights**" means all Patent Rights that (a) are Controlled by Partner or any of its Affiliates as of the Effective Date or during the Term, and (b) Cover the Exploitation of any Licensed Product.

1.130 "**Partner Regulatory Activities**" has the meaning set forth in Section 3.1.1 (Regulatory Activities).

1.131 "**Partner Technology**" means Partner Know-How, Partner Patent Rights, and Partner's interest in the Joint Technology.

1.132 "**Party**" or "**Parties**" has the meaning set forth in the Preamble.

1.133 "**Patent Challenge**" has the meaning set forth in Section 13.2.3 (Termination for Patent Challenge).

1.134 "**Patent Prosecution**" means any activities directed to (a) preparing, filing, and prosecuting applications (of all types) for any Patent Right (including any adversarial proceedings at a patent office related thereto), (b) maintaining any Patent Right (including any adversarial proceedings at a patent office related thereto, such as IPRs, PGRs, re-examinations, oppositions, or equivalent actions), and (c) deciding whether to abandon or maintain any Patent Right.

1.135 "**Patent Rights**" means (a) all patents and patent applications in any country or region, (b) all patent applications filed either from such patents or patent applications or from an application claiming priority from any of these, including divisionals, continuations, continuations-in-part, provisionals, converted provisionals, and continued prosecution applications, (c) any and all patents that have issued or in the future issue from the foregoing patent applications, and (d) any and all substitutions, renewals, registrations, confirmations, extensions, or restorations, including revalidations, reissues, and re-examinations (including any supplementary protection certificates and the like) of the foregoing patents or patent applications.

1.136 "**Person**" means any corporation, limited or general partnership, limited liability company, joint venture, joint stock company, trust, unincorporated association, governmental body, authority, bureau, or agency, or any other entity or body, or an individual.

1.137 [*] has the meaning set forth in Section 10.6.3 [*].

1.138 [*] has the meaning set forth in Section 10.6.3 [*].

1.139 "**Phase III Clinical Trial**" means a clinical trial in humans of a pharmaceutical or biologic product performed to gain evidence with statistical significance of the efficacy of such product in a target population, and to obtain expanded evidence of safety and, if applicable, tolerability for such product that is needed to evaluate the overall benefit-risk relationship of such product, to form the basis for approval of an MAA by a Regulatory Authority and to provide an adequate basis for physician labeling, in a manner that is generally consistent with 21 C.F.R. § 312.21(c), as amended (or its successor regulation), or, with respect to any other country or region, the equivalent of such a clinical trial in such other country or region.

1.140 "**Product Infringement**" has the meaning set forth in Section 12.5.1 (Patent Enforcement; Notice).

1.141 "**Product Labeling**" means prescribing information, patient labeling, and carton and container labeling.

1.142 "**Product Marks**" means any Mark (whether registered or unregistered), other than a uniQure Housemark or a Partner Housemark, for use on, with, or to refer to a Licensed Product or used with patient support or other information or services or Promotional Materials associated with a Licensed Product in the Territory during the Term, together with (a) all registrations, applications for registrations, and other intellectual property rights associated with any of the foregoing, and (b) the goodwill associated with each of the foregoing.

1.143 "**Product Specifications**" has the meaning set forth in the Supply Agreement.

1.144 "**Project Manager**" has the meaning set forth in Section 7.6 (Project Management).

1.145 "**Project Team**" has the meaning set forth in Section 7.6 (Project Management).

1.146 "**Promotional Materials**" means all written, printed, graphic, electronic, audio or video matter, including journal advertisements, sales visual aids, leave behind items, formulary binders, reprints, direct mail, direct-to consumer advertising, Internet postings, broadcast advertisements and sales reminder aids (for example, scratch pads, pens and other like items), in each case, created by a Party or on its behalf and used or intended for use in connection with any promotion of any Licensed Product in the Field in the Territory.

1.147 "**Publication**" has the meaning set forth in Section 9.7 (Publications).

1.148 "**Receiving Party**" has the meaning set forth in Section 9.1.1 (Duty of Confidence).

1.149 "**Regulatory Approval**" means, with respect to a particular country or other regulatory jurisdiction, any approval of an MAA or other approval, product, or establishment license, registration, or authorization of any Regulatory Authority necessary for the commercial marketing, manufacture, importation, exportation or sale of a pharmaceutical or biologic product in such country or other regulatory jurisdiction, excluding, in each case, a Reimbursement Approval.

1.150 "**Regulatory Authority**" means, in a particular country or jurisdiction, any applicable Governmental Authority involved in granting Regulatory Approval or Reimbursement Approval in such country or jurisdiction, including (a) in the E.U., the EMA and any other applicable Governmental Authority in the E.U. having jurisdiction over any pharmaceutical or biologic product, (b) in the U.S., the FDA, the Centers for Medicare & Medicaid Services and any other applicable Governmental Authority in the U.S. having jurisdiction over any pharmaceutical, companion diagnostic, or biologic product, and (c) in other countries, other analogous Governmental Authorities having jurisdiction over any pharmaceutical or biologic product.

1.151 "**Regulatory Exclusivity**" means, [*].

1.152 "**Regulatory Milestone Events**" has the meaning set forth in Section 8.2.1 (Regulatory Milestones).

1.153 "**Regulatory Milestone Payment**" has the meaning set forth in Section 8.2.1 (Regulatory Milestones).

1.154 "**Regulatory Responsible Party**" means Partner; *provided* that, as between the Parties, uniQure will remain the Regulatory Responsible Party to the extent that uniQure is required by Applicable Law or any Regulatory Authority in the Territory [*].

1.155 "**Regulatory Submissions**" means any filing, application, or submission with any Regulatory Authority in support of Developing, Manufacturing, or Commercializing a pharmaceutical or biologic product (including to obtain, support, or maintain Regulatory Approval from that Regulatory Authority), and all substantive correspondence or communication with or from the relevant Regulatory Authority, as well as minutes of any substantive meetings, telephone conferences, or discussions with the relevant Regulatory Authority. Regulatory Submissions include all INDs, MAAs, and other applications for Regulatory Approval and Reimbursement Approvals and each of their equivalents.

1.156 "**Reimbursement Approval**" means an approval, agreement, determination, or other decision by the applicable Governmental Authority that establishes prices charged to end-users for pharmaceutical or biologic products at which a particular pharmaceutical or biologic product will be reimbursed by the Regulatory Authorities or other applicable Governmental Authorities or Persons with contracted arrangements with applicable Regulatory Authorities or Governmental Authorities in the Territory.

1.157 "**Review Period**" has the meaning set forth in Section 9.6 (Publications).

1.158 "**Royalties**" has the meaning set forth in Section 8.3.1 (Royalty Payments).

1.159 "**Royalty Rates**" has the meaning set forth in Section 8.3.1 (Royalty Payments).

1.160 "**Royalty Report**" has the meaning set forth in Section 8.3.3(b) (Royalty Report).

1.161 "**Royalty Term**" has the meaning set forth in Section 8.3.1 (Royalty Payments).

1.162 "**Sales Milestone Events**" has the meaning set forth in Section 8.2.2 (Sales Milestones).

1.163 "**Sales Milestone Payment**" has the meaning set forth in Section 8.2.2 (Sales Milestones).

1.164 "**SDE Agreement**" has the meaning set forth in Section 3.8.2 (SDE Agreements).

1.165 "**Seller**" has the meaning set forth in Section 1.117 (Net Sales).

1.166 "**Specified CMO**" has the meaning set forth in Section 2.2.2 (Right to Subcontract).

1.167 "**Subcontractor**" means a Third Party contractor engaged by a Party or its Affiliates to perform certain services for such Party or its Affiliates under this Agreement on a fee-for-service basis (including CMOs, contract research organizations, contract sales forces or similar Persons).

1.168 "**Sublicensee**" means any Person to whom a Party or its Affiliates grants a sublicense of, or other authorization or permission granted under, the rights granted to a Party under this Agreement (*e.g.*, a Person who has rights to Commercialize the Licensed Product in a particular country in the Territory), excluding all Subcontractors.

1.169 "**Supply Agreement**" has the meaning set forth in Section 5.1 (Supply Agreement).

1.170 "**Tax**" or "**Taxes**" means any present or future taxes, levies, imposts, duties, charges, assessments or fees of any nature (including any interest thereon), including value add taxes ("**VAT**").

1.171 "**Term**" has the meaning set forth in Section 13.1 (Term).

1.172 "**Territory**" means all countries and territories of the world.

1.173 "**Territory Sponsor**" means, with respect to a Clinical Trial for a Licensed Product to be conducted at sites in the Territory, the Party that holds the IND from the applicable Regulatory Authority in the Territory for such Clinical Trial in its name.

1.174 "**Third Party**" means any Person other than a Party or an Affiliate of a Party.

1.175 "**Third Party Claims**" means collectively, any and all Third Party demands, claims, actions, suits, and proceedings (whether criminal or civil, in contract, tort, or otherwise).

1.176 "**Third Party Distributor**" means any Third Party that purchases Licensed Product from Partner or its Affiliates or Sublicensees, takes title to such Licensed Product, and distributes such Licensed Product directly to customers, but does not Develop or Manufacture any Licensed Product and does not make any royalty, profit-share, or other payment to Partner or its Affiliates or Sublicensees, other than payment for the purchase of Licensed Product for resale.

1.177 "**Third Party Royalty Payments**" means, with respect to a Licensed Product, royalty payments made by Partner to a Third Party pursuant to an agreement between Partner and

such Third Party entered into following the Effective Date in consideration for a license to Partner under Patent Rights Controlled by such Third Party that would, but for such license, be infringed by the Exploitation of such Licensed Product in the Territory, including any such payments that accrue or are payable under such agreement after the expiration of any such Patent Rights.

1.178 "**uniQure**" has the meaning set forth in the Preamble.

1.179 "**uniQure Additional Development Activities**" means (a) those Development activities, other than those conducted under the Current Phase III Protocol, performed by uniQure following the Execution Date in accordance with the uniQure Development Budget therefor, and (b) any other Development activities that the JSC determines uniQure will conduct pursuant to Section 4.1.3 (uniQure Additional Development Activities).

1.180 "**uniQure Additional Development Budget**" has the meaning set forth in Section 4.1.3 (uniQure Additional Development Activities).

1.181 "**uniQure Additional Development Plan**" has the meaning set forth in Section 4.1.3 (uniQure Additional Development Activities).

1.182 "**uniQure Development Activities**" means all Development activities performed by or on behalf of uniQure in furtherance of the Current Phase III Protocol and all uniQure Additional Development Activities.

1.183 "**uniQure Development Budget**" has the meaning set forth in Section 4.4.1 (uniQure Development Budget).

1.184 "**uniQure Housemarks**" means (a) the corporate logo of uniQure or any of its Affiliates, (b) the trademark "uniQure," (c) any other Mark (whether registered or unregistered) containing the word "uniQure," (d) any other corporate logo or other Mark used by uniQure to identify uniQure or its Affiliates, (e) all registrations, applications for registrations, and other intellectual property rights associated with any of the foregoing, and (f) all goodwill associated with any and all of the foregoing in clauses (a) through (e).

1.185 "**uniQure Indemnitee(s)**" has the meaning set forth in Section 11.1 (Indemnification; By Partner).

1.186 "**uniQure Interim Development and Manufacturing Development Plan**" has the meaning set forth in Section 10.6.2 (uniQure Additional Covenants Prior to the Effective Date).

1.187 "**uniQure Know-How**" means all Know-How (excluding uniQure's interest in Joint Know-How) that is (a) Controlled by uniQure or any of its Affiliates as of the Effective Date or during the Term, and (b) necessary or reasonably useful to Exploit (i) one or more Licensed Products or (ii) the AAV5 NAb Assay in connection with the Licensed Products, in each case ((i) and (ii)), in the Field in the Territory in accordance with this Agreement. uniQure Know-How includes the uniQure Manufacturing Know-How.

1.188 "**uniQure Manufacturing Know-How**" means all Know-How, other than Joint Know-How, that is (a) Controlled by uniQure or any of its Affiliates as of the Effective Date or during the Term, and (b) related to a step or method of Manufacturing used to Manufacture any Licensed Product.

1.189 "**uniQure Manufacturing Patent Rights**" means all Patent Rights, other than Joint Patent Rights and uniQure Product Patent Rights, that are (a) Controlled by uniQure or any of its Affiliates as of the Effective Date or during the Term, and (b) Cover a step or method of Manufacturing used to Manufacture any Licensed Product.

1.190 "**uniQure Manufacturing Specified Patent Rights**" means any uniQure Patent Right that contains claims that Cover and specifically recite a step or method used to Manufacture a Licensed Product, and do not Cover a step or method of Manufacturing (a) used to Manufacture any product Controlled by uniQure or any of its Affiliates other than a Licensed Product or (b) that may be used generally with Gene Therapy Products other than a Licensed Product.

1.191 "**uniQure Patent Rights**" means all Patent Rights (excluding uniQure's interest in Joint Patent Rights) that are (a) Controlled by uniQure or any of its Affiliates as of the Effective Date or during the Term, and (b) necessary or reasonably useful (or, with respect to patent applications, would be necessary or reasonably useful if such patent applications were to issue as patents) to Exploit (i) one or more Licensed Products or (ii) the AAV5 NAb Assay in connection with the Licensed Products, in each case ((i) and (ii)), in the Field in the Territory in accordance with this Agreement. uniQure Patent Rights include the uniQure Platform Patent Rights, uniQure Product Patent Rights, and uniQure Manufacturing Patent Rights. Schedule 10.2.2 (uniQure Patent Rights) sets forth the uniQure Patent Rights that are owned or exclusively licensed by uniQure or any of its Affiliates in the Territory and that exist as of the Execution Date.

1.192 "**uniQure Platform Patent Rights**" means any uniQure Patent Rights other than the uniQure Product Patent Rights and uniQure Manufacturing Patent Rights. Without limitation, uniQure Platform Patent Rights include Patent Rights that Cover the AAV5 capsid, the LP1 promoter, dosing without screening for preexisting neutralizing antibodies, and redosing.

1.193 "**uniQure Product Patent Rights**" means any uniQure Patent Right that contains claims that (a) Cover and specifically recite (i) a composition of matter that includes the Licensed Product or a transgene for the Padua variant of Factor IX, or (ii) a method of use of any such composition of matter set forth in the foregoing clause (i) and (b) do *not* Cover (A) any product Controlled by uniQure or any of its Affiliates other than a Licensed Product, (B) any component of a Licensed Product that may be used in products that do not deliver the Padua variant of Factor IX, such the AAV5 capsid or the LP1 promoter, or (C) any method of use that may be used with products that do not deliver the Padua variant of Factor IX, such as dosing without screening for preexisting neutralizing antibodies or redosing.

1.194 "**uniQure Royalty Patent Rights**" means [*].

1.195 "**uniQure Technology**" means uniQure Know-How, uniQure Patent Rights, and uniQure's interest in the Joint Technology.

1.196 "**United States**" or "**U.S.**" means the United States of America and its territories and possessions.

1.197 "**Upfront Payment**" has the meaning set forth in Section 8.1 (Upfront Payment).

1.198 "**U.S. Antitrust Filing**" has the meaning set forth in Section 15.2 (Antitrust Filings).

1.199 "**Valid Claim**" means: (a) a claim of an issued and unexpired patent (as may be adjusted through a patent term adjustment or extended through supplementary protection certificate or patent term extension or the like) that has not been revoked, held invalid, or held unenforceable by a patent office or other Governmental Authority of competent jurisdiction in a final and non-appealable judgment (or judgment from which no appeal was taken within the allowable time period); or (b) a pending claim (i) of an unissued, pending patent application within the uniQure Royalty Patent Rights or (ii) claiming priority from a patent or patent application within the uniQure Royalty Patent Rights, *provided* that in each case such pending claim shall cease to be a Valid Claim, [*], unless and until such claim becomes an issued claim of an issued patent, in which case it will again be considered a Valid Claim under the foregoing clause (a) from the date the patent issues and for as long as it meets the requirements of clause (a).

1.200 "**Valid Encryption Process**" has the meaning set forth in Section 3.7 (Security).

1.201 "**Variant**" means an improved or modified product iteration derived from the Lead Product that has or is intended to have pharmacological properties substantially similar to, or superior to, the properties of the composition of the Lead Product, such as codon optimization, an improved capsid, or an improved promoter. "Variant" includes [*] but excludes [*].

1.202 "**VAT**" has the meaning set forth in Section 1.170 (Tax).

1.203 "**Withholding Party**" has the meaning set forth in Section 8.13 (Taxes).

ARTICLE 2
LICENSES

2.1 License Grants to Partner.

2.1.1 **Licensed Products**. Subject to the terms of this Agreement (including uniQure's retained rights set forth in Section 2.4 (Retained Rights)), uniQure hereby grants to Partner an exclusive (even as against uniQure and its Affiliates except as expressly set forth in the last sentence of Section 2.4 (Retained Rights)), royalty-bearing license, with the right to grant sublicenses through multiple tiers solely in accordance with Section 2.2 (Sublicensing and Subcontractors), under the uniQure Technology solely to Exploit the Licensed Products in the Field in the Territory in accordance with this Agreement.

2.1.2 **AAV5 NAb Assay**. Subject to the terms of this Agreement (including uniQure's retained rights set forth in Section 2.4 (Retained Rights)), uniQure hereby grants to Partner an exclusive (even as against uniQure and its Affiliates except as expressly set forth in the last sentence of Section 2.4 (Retained Rights)), fully paid-up, royalty-free license, with the right to grant sublicenses through multiple tiers solely in accordance with Section 2.2 (Sublicensing and Subcontractors), under the uniQure Technology solely to Exploit the AAV5 NAb Assay in connection with the Licensed Products in the Field in the Territory in accordance with this Agreement.

2.2 Sublicensing and Subcontractors.

2.2.1 **Right to Sublicense**. Subject to the terms of this Agreement, Partner will have the right to grant to one or more of its Affiliates, and Partner and its sublicensed Affiliates will have the right to sublicense to its or their respective Subcontractors and Sublicensees sublicenses of the rights granted to Partner under Section 2.1 (License Grants to Partner) without uniQure's prior written consent, except for a sublicense to (i) a Third Party of all or substantially all of Partner's or its Affiliates' rights under this Agreement with respect to one or more Major Countries; or (ii) a CMO that is not one of the Persons set forth on Schedule 2.2.1 (each, a "**Specified CMO**"), if Partner anticipates transferring any uniQure Manufacturing Technology to such CMO, in which case of clause (i) or (ii), the grant of such sublicenses shall require uniQure's prior written consent, which shall not be unreasonably withheld. Any termination of the licenses granted to Partner under Section 2.1 (License Grants to Partner) will cause the permitted Subcontractors or Sublicensees to automatically lose the same rights under any sublicense.

2.2.2 **Right to Subcontract**. Subject to Section 2.2.1 (Right to Sublicense), Section 2.2.4 (Terms of Sublicenses and Subcontracting Agreements), and Section 2.2.6 (Responsibility for Sublicensees and Subcontractors) and the remainder of this Section 2.2.2 (Right to Subcontract), each Party may engage one or more Subcontractors to perform services in furtherance of the performance of its obligations under this Agreement; *provided* that (a) neither Party will engage any such Subcontractor that has been Debarred/Excluded; and (b) no engagement of any such Subcontractors will relieve the engaging Party of its obligations under this Agreement or any liability hereunder. If Partner proposes to engage a CMO to Manufacture any Licensed Product, then Partner will notify uniQure in advance of the identity of such CMO and the activities that Partner proposes such CMO will perform and Partner will consider in good faith any comments of uniQure prior to engaging such CMO, the engagement of which CMO will not require uniQure's consent, except as set forth in Section 2.2.1 (Right to Sublicense).

2.2.3 **Notice of Sublicenses**. Partner will provide uniQure with a true and complete copy of each sublicense agreement with any Subcontractor or Sublicensee no later than [*] after it becomes effective, subject to Partner's right to redact any confidential or proprietary information contained therein that is not necessary for uniQure to (a) determine compliance with the terms of this Agreement or (b) comply with any

obligations under an Existing In-License; *provided* that Partner will only be required to provide uniQure with immaterial Subcontracts to the extent that uniQure is required to provide such immaterial Subcontracts to its licensor under any Existing In-License.

2.2.4 **Terms of Sublicenses and Subcontracting Agreements**. Each sublicense granted by Partner and each subcontracting agreement entered into by either Party pursuant to Section 2.2.1 (Right to Sublicense) or Section 2.2.2 (Right to Subcontract), as applicable, will (a) be subject and subordinate to this Agreement, (b) be consistent with the applicable terms of this Agreement, (c) include obligations of confidentiality and non-use applicable to the Confidential Information of the other Party that are at least as stringent as those set forth in Article 9 (Confidentiality; Publication), (d) include terms that are consistent with the intellectual property provisions set forth in this Agreement, including an assignment or license (sublicensable through multiple tiers) back to the subcontracting or sublicensing Party of all Patent Rights and Know-How necessary or reasonably useful to Exploit any Licensed Product in the Field in the Territory (such that such Party Controls such Patent Rights and Know-How for the purposes of this Agreement), and (e) for a sublicense of Partner's Commercialization rights to Sublicensees, include a provision giving uniQure the right to enforce the applicable terms of such sublicense directly against the Sublicensee, effective after [*] written notice to Partner, in the event that the Sublicensee breaches the sublicense in a manner that harms uniQure, and Partner does not cause the Sublicensee to cure such breach or terminate the applicable sublicense within the deadlines set forth in the applicable sublicense agreement.

2.2.5 **Reports of Sublicensees and Subcontractors**. Each Party will provide the other Party with all information that is reasonably necessary for the other Party to confirm compliance with this Agreement by any Sublicensees or Subcontractors no later than [*] in advance of each JSC meeting (or otherwise upon a Party's request, if reasonably justified under the circumstances).

2.2.6 **Responsibility for Sublicensees and Subcontractors**. Each Party will require that all Sublicensees and Subcontractors perform the activities that they are sublicensed or engaged to perform (as applicable) in accordance with GLP, cGMP, and GCP, as applicable, and otherwise in compliance with Applicable Law. Notwithstanding the grant of any sublicense or engagement of any Subcontractor, each Party will remain primarily liable to the other Party for the performance of such Party's obligations under, and such Party's compliance with all provisions of, this Agreement. Each Party will be fully responsible and liable for any breach of the terms of this Agreement by any of its Sublicensees or Subcontractors to the same extent as if such Party itself has committed any such breach (*provided* that a Party may not recover twice against the other Party and such Sublicensees or Subcontractors for the same breach) and will terminate promptly the agreement with any Sublicensee or Subcontractor if such Sublicensee or Subcontractor Defaults under this Agreement in a manner that materially harms the other Party and does not cure such Default in a timely manner.

2.3 License Grants to uniQure.

2.3.1 **Partner Technology License**. Subject to the terms of this Agreement (including the exclusive licenses granted to Partner hereunder), Partner hereby grants to uniQure a non-exclusive royalty-free license (with the right to grant sublicenses through multiple tiers, subject to the use restrictions below and in Section 2.2 (Sublicensing and Subcontractors)), under the Partner Technology for use solely to the extent necessary to perform uniQure's obligations under this Agreement.

2.3.2 **Manufacturing Technology License**. Subject to the terms of this Agreement, Partner hereby grants and agrees to grant to uniQure a royalty-free, fully paid-up, worldwide, non-exclusive, perpetual, irrevocable license (with the right to grant sublicenses through multiple tiers) under the Partner Manufacturing Technology for use solely in connection uniQure's proprietary manufacturing process [*] (and not for standalone use) (a) to perform uniQure's obligations under this Agreement and (b) for any other purpose, other than to Exploit any Licensed Product or Competitive Product in the Field in the Territory.

2.4 Retained Rights. Nothing in this Agreement will be interpreted to grant Partner any rights under any intellectual property rights owned or Controlled by uniQure, including uniQure Technology, that are not expressly granted herein, whether by implication, estoppel, or otherwise. Any rights not expressly granted to Partner by uniQure under this Agreement are hereby retained by uniQure. In addition, notwithstanding the exclusive license in Section 2.1 (License Grants to Partner) or any other provision to the contrary set forth in this Agreement, uniQure and its Affiliates expressly retain the right to (a) Manufacture the Licensed Products in the Territory for Partner hereunder and (b) perform the uniQure Development Activities and uniQure's other obligations under this Agreement.

2.5 Existing In-Licenses. Notwithstanding any provision to the contrary set forth in this Agreement, Partner or uniQure (as applicable) stipulates and agrees that (a) the rights and licenses granted to Partner under this Agreement are subject to the applicable terms of all Existing In-Licenses with respect to the uniQure Technology that is being sublicensed thereunder, to the extent that such terms have been provided to Partner prior to the Execution Date in an unredacted copy of the applicable Existing In-License (but, for clarity, Partner is not assuming any of uniQure's obligations under any Existing In-License, and uniQure remains solely liable for all fees, royalties, and other obligations thereunder), (b) uniQure's ability to comply with its obligations to Partner under this Agreement is subject to the requirements and restrictions imposed on uniQure under the Existing In-Licenses (to the extent provided by uniQure to Partner in unredacted form) with respect to the uniQure Technology that is being sublicensed under such Existing In-Licenses, (c) uniQure will not be required to take any action or inaction that would cause uniQure to be in breach of any Existing In-License to the extent the terms thereof were provided to Partner in an unredacted form prior to the Execution Date, (d) uniQure shall take those actions and exercise those rights under the Existing In-Licenses (including rights arising in a bankruptcy of any party thereto) as necessary to protect and maintain Partner's rights under this Agreement, (e) uniQure shall not terminate (or take any action to cause the termination of) or fail to renew any Existing In-License without Partner's prior written

consent, (f) uniQure will not amend any Existing in-License in any manner that materially harms Partner's rights herein without Partner's prior written consent, and (g) uniQure will promptly notify Partner of any breach by any party to any Existing In-License and, if uniQure fails to timely cure any such breach by uniQure, will cooperate to allow Partner to cure such breach prior to the expiration of the applicable cure period.

2.6 **Exclusivity Covenant**. Each Party covenants and agrees that neither it nor any of its Affiliates (including, in the case of uniQure, Affiliates of uniQure NV and, in the case of Partner, Affiliates of CSL Limited) will, either alone or in collaboration with (including pursuant to a license from or to) any Third Party, (a) submit any IND for, or, in the event that the submission of an IND is not required by Applicable Law in a particular country or jurisdiction, begin dosing any patient in such country or jurisdiction in any clinical trial for, any Competitive Product during the period commencing as of the Execution Date and continuing until [*], or (b) Commercialize any Competitive Product during the period commencing as of the Execution Date and continuing until [*]. Notwithstanding anything to the contrary set forth in this Agreement, in the event that uniQure is acquired by a Third Party and such Third Party owns or controls a Competitive Product, the further Exploitation of such Competitive Product (or commencement of Exploitation of a Competitive Product) by such Third Party acquirer or its Affiliates after the consummations of the applicable acquisition transaction will not constitute a violation of this Section 2.6 (Exclusivity Covenant) so long as (i) such Third Party acquirer and its Affiliates shall not use or have access to any Confidential Information of Partner (whether relating to the clinical Development or Commercialization of any Licensed Products or otherwise) and (ii) no personnel of such Third Party acquirer and its Affiliates involved in performing clinical Development or Commercialization of such Competitive Product shall use or have access to any uniQure Technology.

ARTICLE 3
REGULATORY

3.1 **Regulatory Responsibilities**.

3.1.1 **Regulatory Activities**. Except as contemplated by Section 3.1.2 (Partner Requests) and Section 3.1.3 (Required by Law) and as set forth in Section 3.2 (Preparation of the Initial U.S. MAA and the Initial E.U. MAA) and Section 3.3 (Other Regulatory Submissions), and the Partner Development Plan, Partner shall have sole authority, control, and decision-making authority with respect to all regulatory activities, filings, MAAs, and other Regulatory Submissions. Partner, itself or through its designee, at its sole cost and expense, will be the Regulatory Responsible Party and accordingly will be solely responsible for, and have decision-making authority with respect to all regulatory activities with respect to the Licensed Products in the Territory, including the filings of all MAAs, applications for Reimbursement Approvals, and other Regulatory Submissions and in relation to all activities required to obtain, support, or maintain Regulatory Approval or Reimbursement Approval for the Licensed Products in the Territory, except as contemplated by Section 3.1.2 (Partner Requests) and Section 3.1.3 (Required by Law) (the "**Partner Regulatory Activities**").

3.1.2 **Partner Requests**. Partner will have sole authority, control, and decision-making authority with respect to MAAs and Regulatory Submissions, but may request uniQure's support for the preparation of any MAA or Regulatory Submissions related thereto or for seeking and maintaining Regulatory Approvals or Reimbursement Approvals for the Licensed Products.

3.1.3 **Required by Law**. Partner will have sole authority, control, and decision-making power with respect to MAAs and Regulatory Submissions, but if uniQure is required by Applicable Law or Regulatory Authorities in the Territory [*], then uniQure will remain the Regulatory Responsible Party with respect to each of the activities set forth in the foregoing clauses (a) and (b).

3.2 Preparation of the Initial U.S. MAA and the Initial E.U. MAA.

3.2.1 **Collaboration**. The Parties' regulatory teams will collaborate to prepare the first MAA for a Licensed Product to be submitted to the FDA in the U.S. (the "**Initial U.S. MAA**"), the first MAA for a Licensed Product to be submitted to the EMA (the "**Initial E.U. MAA**"), and all Regulatory Submissions related thereto, but Partner shall have sole decision-making authority over and control of all Regulatory Submissions. If any such Regulatory Submission is due during the regulatory transfer period described in Section 3.5.1 (Regulatory Transfer), or if uniQure is required to be the Regulatory Responsible Party for any such Regulatory Submission, then Partner shall direct, oversee, review, and approve any Regulatory Submission by uniQure, and uniQure shall act in accordance with Partner's reasonable direction, and uniQure shall transfer and assign to Partner all rights, title, and interests in and to such Regulatory Submission and any Regulatory Approvals and Reimbursement Approvals.

3.2.2 **Cooperation**. To the extent requested by Partner, uniQure shall cooperate with Partner, review any content that Partner asks uniQure to review, and prioritize actions to support Partner in the preparation, review, submission, and maintenance of the Initial U.S. MAA, the Initial E.U. MAA, all Regulatory Submissions related thereto, and seeking and maintaining Regulatory Approval and Reimbursement Approval.

(a) **Initial U.S. MAA**. Partner will (a) collaborate with uniQure on the preparation of the Manufacturing portion of the Initial U.S. MAA, and (b) submit the draft Initial U.S. MAA to the relevant subject matter experts at uniQure for their review and comment sufficiently in advance of any deadlines related to the filing of the Initial U.S. MAA with the FDA, *provided* that Partner may provide the draft Initial U.S. MAA to uniQure in sections. The relevant subject matter experts at uniQure will provide comments on the sections of the draft Initial U.S. MAA to Partner in accordance with any deadlines reasonably set by Partner, but in any event uniQure will have not less than [*], to review each section of the draft Initial U.S. MAA, and Partner will reasonably consider and address in good faith uniQure's comments thereon. To the extent practicable, Partner will

provide uniQure with reasonable advanced notice of its anticipated delivery to uniQure of any such section for uniQure's review and comment.

(b) **Initial E.U. MAA**. Partner will submit the content in the draft Initial E.U. MAA that is substantively different from the content that uniQure had a right to review for the Initial U.S. MAA and any other content that Partner requests uniQure to review to the relevant subject matter experts at uniQure for their review and comment sufficiently in advance of any deadlines related to the filing of the Initial E.U. MAA with the EMA, *provided* that Partner may provide such content to uniQure in sections. If the content that is substantively different from the Initial U.S. MAA includes Manufacturing content and uniQure is then Manufacturing the Licensed Products, then Partner will collaborate with uniQure on the preparation of the applicable Manufacturing content. The relevant subject matter experts at uniQure will provide comments on such content to Partner in accordance with any deadlines reasonably set by Partner, but in any event uniQure will have not less than [*], to review each section of the Initial E.U. MAA that contains content that is substantively different from the content that uniQure had a right to review for in the Initial U.S. MAA, and Partner will reasonably consider and address in good faith uniQure's comments thereon. To the extent practicable, Partner will provide uniQure with reasonable advanced notice of its anticipated delivery to uniQure of any such content for uniQure's review and comment.

3.2.3 **Reimbursement of Costs and Expenses**. Partner will reimburse all reasonable out-of-pocket Third Party expenses and reasonable, documented internal costs (at the FTE Rate) incurred by uniQure after the Effective Date in connection with the activities described in Section 3.2.2 (Cooperation), including uniQure's preparation of the Manufacturing portions to be included in the Initial U.S. MAA and Initial E.U. MAA (*provided* that Partner's reimbursement of any such costs and expenses related to uniQure's preparation of the Manufacturing portions to be included in the Initial U.S. MAA and Initial E.U. MAA will not exceed [*]) and such other activities performed at Partner's request, but expressly excluding any costs and expenses incurred by uniQure for uniQure's review of and comment on the Initial U.S. MAA or the content in the Initial E.U. MAA (other than with respect to the Manufacturing sections thereof).

3.3 Other Regulatory Submissions.

3.3.1 **Preparation of Regulatory Submissions.** Subject to the terms of this Agreement, and except as expressly set forth in Section 3.2 (Preparation of the Initial U.S. MAA and the Initial E.U. MAA), the Regulatory Responsible Party will be responsible for the preparation and submission of all Regulatory Submissions for the Licensed Products in the Field in the Territory. If uniQure is the Regulatory Responsible Party, then uniQure: (a) will be subject to Partner's direction, oversight, review, and approval of all Regulatory Submissions and uniQure shall act in accordance with Partner's reasonable direction; (b) will provide Partner with an opportunity to

review and comment on all Regulatory Submissions to be submitted to any Regulatory Authority in the Territory by or on behalf of the Regulatory Responsible Party for the Licensed Products in the Field in the Territory far enough in advance so as to meet the applicable submission or response deadline for such Regulatory Submission; and (c) will, and will cause its Affiliates and Sublicensees to, implement all reasonable comments thereon from Partner. If (a) Partner is the Regulatory Responsible Party, (b) uniQure is then Manufacturing the applicable Licensed Product, and (c) Partner has made a Material Change to the Manufacturing Section in a Regulatory Submission other than the Initial U.S. MAA or the Initial E.U. MAA, then Partner will provide the relevant subject matter experts at uniQure with an opportunity to review and comment on a Material Change to the Manufacturing Section to be submitted to any Regulatory Authority in the Territory by or on behalf of Partner for the Licensed Products in the Field in the Territory sufficiently in advance of any deadlines related to such Regulatory Submission. The relevant subject matter experts at uniQure will provide comments on such Material Change to the Manufacturing Section to Partner in accordance with any deadlines reasonably set by Partner, but in any event uniQure will have not less than [*] to review each Material Change to the Manufacturing Section, and Partner will reasonably consider and address in good faith uniQure's comments thereon. A "**Material Change to the Manufacturing Section**" means a modification that changes the Manufacturing content substantively, but does not include a formatting change or a change that would provide more or less detail. In addition, Partner may request, and uniQure and its relevant subject matter experts will provide if so requested, new or additional information, comments, or review of any content that Partner requests that uniQure review. The relevant subject matter experts at uniQure will provide comments on a Material Change to the Manufacturing Section, and if so requested such other information, comments or review, to Partner in accordance with any deadlines set by Partner, and Partner will reasonably consider and address in good faith uniQure's comments on the Material Change to the Manufacturing Section.

3.3.2 **Reimbursement of Costs and Expenses.** Partner will reimburse all reasonable out-of-pocket Third Party expenses and reasonable, documented internal costs (at the FTE Rate) incurred by uniQure after the Effective Date at Partner's request in connection with the activities described in Section 3.3.1 (Preparation of Regulatory Submissions).

3.4 Correspondence and Meetings with Regulatory Authorities and Regulatory Approvals.

3.4.1 **Correspondence with Regulatory Authorities**. Except as otherwise set forth in this Agreement, and without limiting Section 3.2 (Preparation of the Initial U.S. MAA and the Initial E.U. MAA) or Section 3.3 (Other Regulatory Submissions), promptly following the Regulatory Responsible Party's receipt, forwarding, or production thereof, the Regulatory Responsible Party will provide the other Party with (a) access to or copies of all material written or electronic correspondence and communications (other than Regulatory Submissions) received by the Regulatory

Responsible Party or its Affiliates or Sublicensees from, or forwarded by the Regulatory Responsible Party or its Affiliates or Sublicensees to, the Regulatory Authorities in the Territory related to the Licensed Product, and (b) copies of all meeting minutes and summaries of all meetings, conferences, and discussions held by the Regulatory Responsible Party or its Affiliates or Sublicensees with the Regulatory Authorities related to the Licensed Products in the Territory, *provided* that, when Partner is the Regulatory Responsible Party, Partner is only obligated to provide uniQure with access to or copies of the correspondence and information in clause (a) or clause (b) pertaining to (i) uniQure's Manufacture of the Licensed Products if uniQure is then Manufacturing the Licensed Products, (ii) safety changes, or (iii) material changes to a Licensed Product's Product Labeling that pertain to new efficacy claims or patient populations, *provided* that Partner will only be obligated to provide the information in sub-clause (iii) for [*] after the date of receipt of the first Regulatory Approval in the United States. Partner, in its sole discretion, may provide uniQure with access to or copies of the correspondence and information in clause (a) or clause (b) if such correspondence or information requires uniQure's support, including information to be provided by uniQure or support with respect to any Regulatory Submission, Regulatory Approval, Reimbursement Approval, or uniQure's Manufacturing or chemistry, manufacturing, and control activities. If such written or electronic correspondence received from any such Regulatory Authority relates to the prohibition or suspension of the supply of the Licensed Product, or the initiation of any investigation, review, or inquiry by such Regulatory Authority concerning the safety of the Licensed Product, then, if uniQure is the Regulatory Responsible Party, the Regulatory Responsible Party will notify the other Party and provide the other Party with copies of such written or electronic correspondence as soon as reasonably practicable. If Partner is the Regulatory Responsible Party, uniQure is still Manufacturing the applicable Licensed Product, and such written or electronic correspondence relates to the Manufacture or safety of the Licensed Product, then Partner will notify uniQure, and provide uniQure with copies of such written or electronic correspondence as soon as reasonably practicable, but not later than [*] after receipt of such correspondence.

3.4.2 **Meetings with Regulatory Authorities**. Except as otherwise set forth in this Agreement, the applicable Regulatory Responsible Party will be responsible for all meetings, conferences, and discussions with Regulatory Authorities or other applicable Governmental Authorities related to the receipt of Regulatory Approval and Reimbursement Approval to Commercialize the Licensed Products in the Field in the Territory. If uniQure is the Regulatory Responsible Party, then uniQure will provide Partner with written notice of any scheduled material meeting, conference, or discussion with a Regulatory Authority or other Governmental Authority in the Territory relating to any Licensed Product in the Field as soon as practicable and, upon such other Party's written request, will, to the extent practicable and permitted by the Governmental Authority in the Territory, allow such other Party to attend such meeting, conference, or discussion. If Partner is the Regulatory Responsible Party, then Partner has sole discretion to allow uniQure to attend such meetings, conferences, or discussions, and will consider uniQure's requests to attend in good

faith, if any of the following topics are scheduled for discussion: data generated by uniQure that is required for receipt of Regulatory Approval, uniQure's Manufacturing of the Licensed Products, uniQure's chemistry, manufacturing, and control activities for the Licensed Products, or uniQure's support for the approval of the Initial U.S. MAA or the Initial E.U. MAA, *provided* that Partner may limit the number of participants from uniQure to one subject matter expert per topic. If Partner is the Regulatory Responsible Party, then Partner may also reasonably request that uniQure attend such meetings, conferences, or discussions. The Regulatory Responsible Party will provide to the other Party copies of any correspondence relating to such meetings, conferences, or discussions, including meeting requests, briefing materials or questions as soon as reasonably practicable, and in any case, no later than [*] after the Regulatory Responsible Party's receipt thereof, *provided* that if Partner is the Regulatory Responsible Party, then Partner will only provide such copies if uniQure was a participant in such meetings, conferences, or discussions.

3.4.3 **Ownership of Regulatory Approvals**. The Regulatory Responsible Party will file all MAAs and applications for Reimbursement Approval for the Licensed Products in the Field in the Territory in its name, *provided* that if Partner is the Regulatory Responsible Party, Partner may file all such MAAs and applications in the name of Partner's Affiliates or Third Party Distributors, and, subject to the assignments set forth in Section 3.5 (Transfer of Regulatory Materials) and the rights granted to Partner under Section 2.1 (License Grants to Partner), will own all rights, title, and interests in and to all resulting Regulatory Approvals and Reimbursement Approvals for the Licensed Products in the Field in the Territory and all related Regulatory Submissions. The Regulatory Responsible Party will promptly inform the other Party (and in any event no later than [*] after receipt) of (a) the filing of any MAA for any Licensed Product in the Field in the Territory, and (b) the receipt of any Regulatory Approval or Reimbursement Approval for any Licensed Product in the Field in the Territory.

3.5 Transfer of Regulatory Materials.

3.5.1 **Regulatory Transfer**. No later than [*] following the database lock for clinical data under the Current Phase III Protocol uniQure will, or will cause its designee to, transfer and assign to Partner all rights, title, and interests in and to all Regulatory Approvals, Reimbursement Approvals, and all other Regulatory Submissions required under Applicable Law in the Territory to be held by Partner related to Commercialization or Development of the Licensed Product in the Field in the Territory in the possession and Control of uniQure, its Affiliates, or designees (the "**Assigned Regulatory Materials**"), including copies of all such Assigned Regulatory Materials in electronic format, or such other format maintained by uniQure or its designee or otherwise agreed by the Parties. uniQure shall promptly, but no later than [*] after the Effective Date, provide Complete Regulatory Files for all interactions with any Regulatory Authorities or Governmental Authorities in the Territory, and a catalogue of the Complete Regulatory Files, in a format that is suitable for Partner's electronic storage system. "**Complete Regulatory Files**"

means (a) each individual sequence submitted in its entirety, unaltered from the original submission, including: (i) full folder structure of each individual sequence, (ii) portable document format ("**PDF**") all documents submitted within that sequence, and (iii) all extensible markup language ("**XML**") documents submitted in that sequence; (b) documentation submitted to or received from, and oral or written correspondence with, any Regulatory Authorities or Governmental Authorities related to the Licensed Products and any companion diagnostic, including meeting minutes, notes pertaining to formal or informal discussions, electronic mail correspondence, telephone conversation records, Regulatory Submissions, and communications related to Clinical Trials, Development, and Manufacturing, and (c) all technical information relating to the Licensed Products that uniQure has in its possession and that is required to be submitted to a Regulatory Authority for the purpose of obtaining any Regulatory Approval of the Licensed Products. The Parties will review and discuss the detailed timeline and each Party's roles and responsibilities through the JSC and will consult with applicable Regulatory Authorities regarding such assignment and transfer sufficiently in advance thereof.

3.5.2 **Cooperation**. Subject to the terms of this Agreement, upon Partner's written request, uniQure will execute and deliver, or will cause to be executed and delivered, to Partner such endorsements, assignments, commitments, acknowledgements, and other documents as may be necessary (a) to assign, convey, transfer, and deliver to Partner all of uniQure's or its applicable Affiliate's or designee's rights, title, and interests in and to the applicable Assigned Regulatory Materials, (b) to otherwise assume the responsibilities as the Regulatory Responsible Party under this Agreement, or (c) as a result of the transfer to Partner of the Assigned Regulatory Materials, including submitting to each applicable Regulatory Authority or other Governmental Authority in the Territory a letter or other necessary documentation (with copy to Partner) notifying such Regulatory Authority or other Governmental Authority of, or otherwise giving effect to, the transfer of ownership to Partner of the Assigned Regulatory Materials, Reimbursement Approvals, and other Regulatory Submissions for any Licensed Product in the Field in the Territory as provided in Section 3.5.1 (Regulatory Transfer).

3.5.3 **Costs**. Partner will bear all reasonable out-of-pocket Third Party expenses in connection with (a) the transfer and assignment of all Assigned Regulatory Materials, and any other copies of any Regulatory Approvals, Reimbursement Approvals, or other Regulatory Submissions for any Licensed Product in the Field in the Territory provided to Partner pursuant to this Section 3.5 (Transfer of Regulatory Materials), and (b) the performance of any other activities required for Partner to assume the role of Regulatory Responsible Party with respect to any Licensed Product in the Field in the Territory or as may be required as a result of the transfer to Partner of the Assigned Regulatory Materials, *provided* that Partner's reimbursement of any such expenses will not exceed [*]. Accordingly, uniQure will invoice Partner for such costs and expenses incurred by or on behalf of uniQure in connection with the performance of such transfer and activities related thereto, and

Partner will pay the undisputed invoiced amounts within [*] after the date of the invoice.

3.6 **Regulatory Data Requests**. Subject to the terms of this Agreement, uniQure may request from Partner data on [*], in each case, that is included in Regulatory Submissions pertaining to any Licensed Product in the Field in the Territory submitted by or on behalf of Partner or its Affiliates, for use in the Regulatory Submissions for any product Controlled by uniQure or its Affiliates that contains the same capsid, promoter, or other component that is contained in the Licensed Product, but excluding, for the avoidance of doubt, any Competitive Product. Partner will consider each request to provide or use such data, as applicable, on a case-by-case basis and will not unreasonably withhold or delay its provision of such data to, or the use of such data by, uniQure.

3.7 **Security**. The Parties will send and transfer all regulatory materials discussed in Article 3 (Regulatory), including draft Regulatory Submissions, correspondence with Regulatory Authorities or Governmental Authorities, and copies of meeting minutes, in each case, using a Valid Encryption Process for data at rest or data in motion, as applicable. "**Valid Encryption Processes**" are those that comply with National Institute of Standards and Technology (NIST) Special Publications, including 800-111, Guide to Storage Encryption Technologies for End User Devices; 800-52, Guidelines for the Selection and Use of Transport Layer Security (TLS) Implementations; 800-77, Guide to IPsec VPNs; or 800-113, Guide to SSL VPNs, or are Federal Information Processing Standards (FIPS) 140-2 validated, or provide a stronger level of data security than contemplated by such Special Publications or Standards or as may be otherwise agreed by the Parties in writing. Promptly following the Effective Date, the Parties will discuss and agree upon a Valid Encryption Process for such transfer in compliance with the requirements of this Section 3.7 (Security).

3.8 **Adverse Events Reporting**.

3.8.1 **Adverse Event Reporting**. Within a reasonable period of time prior to the First Commercial Sale of the first Licensed Product in the Field in the Territory, the Parties will notify each other in writing of the names and contact information of their respective employees or agents who are responsible for adverse experience reporting.

3.8.2 **Safety Data Exchange (SDE) Agreements**. No later than [*] after the Effective Date, the Parties will enter into one or more written agreements setting forth safety and pharmacovigilance procedures for the Parties with respect to the Licensed Products in the Field in the Territory (a "**SDE Agreement**"). Partner will not market, promote, sell, or otherwise Commercialize any Licensed Product unless and until the Parties enter into one or more SDE Agreements for the Licensed Product. Each SDE Agreement will describe the obligations of both Parties with respect to the collection, investigation, reporting, and exchange of information between the Parties concerning any adverse event experienced by a subject or patient, and the seriousness thereof, whether or not determined to be attributable to a Licensed Product, including any such information received by either Party from a Third Party (subject to receipt of any required consents from such Third Party)

and will be sufficient to permit each Party and its Affiliates, licensees, or Sublicensees (as applicable) to comply with its legal obligations with respect thereto, including each Party's obligations as the owner or holder of Regulatory Approvals and Regulatory Submissions (including INDs) for such Licensed Product in the Territory, as applicable. Each SDE Agreement will also detail (a) each Party's responsibilities with respect to the maintenance of a safety database, (b) the other Party's rights to access and query such database, it being understood that (x) each Party can run safety data queries for the other Party, *provided* that such safety data queries are limited to (i) safety questions on the vector from Regulatory Authorities in order to respond to ad hoc queries from Regulatory Authorities, and (ii) four queries per Party per year, and *further provided* that the Parties must reach an agreement regarding any interpretations and conclusions to be submitted to the Regulatory Authorities, and (y) uniQure will maintain the global safety database for the Licensed Products until the primary and secondary endpoints have been reached (which is estimated to be [*]), at which time uniQure will transition to Partner such global safety database and thereafter Partner will run the safety data queries described in clause (x) in such global safety database on reasonable notice at uniQure's request up to four times per year, *provided* that the queries will be limited to safety questions on the vector from Regulatory Authorities in order to respond to ad hoc queries from Regulatory Authorities, and *further provided* that Partner will run such queries using a methodology to be set out in the applicable SDE Agreement, (c) each Party's responsibilities for recalls and withdrawals of any Licensed Product in the Territory, and (d) uniQure's obligation to run safety data queries on reasonable notice at Partner's request, *provided* that the queries will be limited to safety questions on the vector from Regulatory Authorities, the queries may be run on safety data collected by uniQure from products other than the Lead Product or the Licensed Product, and the queries will be limited to four times per year. In the SDE Agreement, the Parties, will set forth the scope and time frame for the queries in clauses (b) and (d), parameters for discussions regarding the interpretations and conclusions reached from such queries prior to the submission of the queries and the interpretations and conclusions to a Regulatory Authority, and the reciprocal incorporation of the queries and interpretations and conclusions into the current periodic report of each Party (*e.g.*, DSUR, PSUR), a copy of which is provided to the other Party. If the Parties, do not reach an agreement regarding any interpretations and conclusions to be submitted to the Regulatory Authorities, as described in clauses (b) and (d), then Partner will have the final decision-making authority regarding such interpretations and conclusions for Licensed Products and uniQure will have the final decision-making authority regarding such interpretations and conclusions for products other than the Licensed Products. If required by changes in Applicable Law, the Parties will make appropriate updates to the applicable SDE Agreements. Each Party will comply with its respective obligations under each SDE Agreement and cause its Affiliates and Sublicensees to comply with such obligations. Notwithstanding any provision to the contrary in this Agreement or any SDE Agreement, each Party and its Affiliates, licensees, and Sublicensees will have the right to disclose information related to the safety of any Licensed Product to the extent that such disclosure is required for such Party to

comply with its obligations under Applicable Law or the safety requirements of the applicable Regulatory Authorities. The Parties will cooperate with each other to address any safety-related inquiries or requests for safety assessment by any Regulatory Authority, including providing any necessary data or information in a timely manner. To the extent that there is a conflict between the terms of this Agreement and the terms of any SDE Agreement, the terms of the applicable SDE Agreement will govern with respect to the subject matter set forth therein.

3.9 **Notice of Regulatory Actions**. In addition, each Party will promptly notify the other Party of any information that such Party receives regarding any threatened or pending action, inspection, or communication by or from a Regulatory Authority that would reasonably be expected to materially affect the Exploitation of any Licensed Product in the Field in the Territory.

ARTICLE 4
DEVELOPMENT PROGRAM

4.1 **Development**.

4.1.1 **General**. Without limiting Partner's responsibility for the Partner Regulatory Activities, which will be conducted by or on behalf of Partner for the Licensed Products in accordance with Section 3.1 (Regulatory Responsibilities) and, except for any uniQure Development Activities, Partner, at its sole cost and expense and subject to the terms of this Agreement, will be solely responsible for all Development activities for the Licensed Products in the Territory.

4.1.2 **Development Diligence**. Subject to the terms of this Agreement, uniQure will use Commercially Reasonable Efforts to (a) conduct the Development of any Licensed Product in accordance with the Current Phase III Protocol (as such protocol may be updated from time to time pursuant to Section 4.4.1 (Current Phase III Protocol and Budget) and (b) perform any other uniQure Development Activities, including pursuant to the uniQure Development Budget or any uniQure Additional Development Plan (as such plan may be updated from time to time pursuant to Section 4.4.3 (uniQure Additional Development Plan and Budget). Partner will use Commercially Reasonable Efforts to (i) obtain and maintain, and (ii) perform all Development activities required or recommended to support, obtain, and maintain, in each case ((i) and (ii)), all Regulatory Approvals and Reimbursement Approvals for each Licensed Product in the Field in each Major Country necessary to Commercialize the Licensed Product in such Major Country, including any Development activities required or recommended as a result of a change to one or more Manufacturing processes for a Licensed Product.

4.1.3 **uniQure Additional Development Activities**. If Partner desires that uniQure perform any Development activities for any Licensed Product (other than those set forth in the Current Phase III Protocol, the Manufacturing Development Plan, or relating to CMC development activities related to GMP production of a Licensed Product), then Partner will present to the JSC a proposal that outlines such activities

and the costs and expenses associated therewith. The JSC will review, discuss, and determine in accordance with Article 7 (Governance) whether to approve uniQure's performance of any such Development activities and a written plan of such Development activities to be performed by uniQure, including a plan for reporting on Development activities thereunder and a budget of all costs and expenses to be incurred in connection with the performance of such Development activities by uniQure (any such Development activities that the JSC determines uniQure will conduct will be uniQure Additional Development Activities, and the plan approved by the JSC pursuant to which any such uniQure Additional Development Activities will be conducted, as such plan be updated pursuant to Section 4.4.3 (uniQure Additional Development Plan and Budget), the "**uniQure Additional Development Plan**," and the budget approved by the JSC of the costs and expenses to be incurred in connection with the performance of any uniQure Additional Development Activities, as such budget may be updated pursuant to Section 4.4.3 (uniQure Additional Development Plan and Budget), the "**uniQure Additional Development Budget**"). Partner will reimburse uniQure for any uniQure Additional Development Activities in accordance with Section 4.4.4 (Allocation of Costs).

4.2 **Partner Development Plan**. No later than [*] after the Effective Date, Partner will develop an initial draft of the written plan for Development of the Licensed Products in the Field in the Territory (each, as updated from time to time in accordance with this Section 4.2 (Partner Development Plan) and Section 7.2 (Joint Steering Committee), the "**Partner Development Plan**") and provide such initial draft to the JSC to review and discuss. The Partner Development Plan must include in reasonable detail: [*]. Partner will prepare an update to each Partner Development Plan at least annually for each Financial Year and will submit each such updated Partner Development Plan to the JSC to review and discuss.

4.3 **Development Reporting**. At least [*] in advance of each JSC meeting, Partner will provide the JSC with a written report, in reasonable detail, regarding Partner's Development activities by Financial Quarter with respect to the Licensed Products, which report will identify all Third Parties performing material Development activities, the key Development activities to be undertaken in the upcoming Financial Year throughout the Territory, and planned dates of submission of any MAA for a Licensed Product and receipt of Regulatory Approval for a Licensed Product. uniQure will: (a) [*],provide Partner with a written report, in reasonable detail, regarding uniQure Development Activities conducted under the Current Phase III Protocol, which report will describe such uniQure Development Activities conducted by uniQure in the prior [*] and to be undertaken in the upcoming [*]; (b) participate in status calls with Partner, such calls to be scheduled at a mutually agreeable time between the Parties from time to time (but not more frequently than once per [*]); (c) notify Partner of the occurrence of (together with reasonable details of) any event that has, or may reasonably be likely to have, any serious adverse effect relating to Development of the Licensed Products within [*] of uniQure becoming aware of such event; and (d) upon receipt of any written request from Partner (which requests may not be made more frequently than [*]), uniQure will provide Partner with a written response to any reasonable information request with respect to uniQure's Development Activities.

4.4 uniQure Development Plans and Budgets.

4.4.1 **uniQure Development Budget**. Schedule 4.4.1 (uniQure Development Budget) sets forth the complete budget for all uniQure Development Activities for the Licensed Product to be conducted by uniQure following the Effective Date, including for the conduct of the Current Phase III Protocol (and associated Current Phase III Budget) and any uniQure Additional Development Activities (and associated budget therefor) contemplated as of the Execution Date (the "**uniQure Development Budget**"). uniQure will update the uniQure Development Budget to reflect (a) any updates to the Current Phase III Budget pursuant to Section 4.4.2 (Current Phase III Protocol and Budget) and (b) any uniQure Additional Development Budget (or any update thereto) approved by the JSC pursuant to Section 4.1.3 (uniQure Additional Development Activities) or Section 4.4.3 (uniQure Additional Development Plan and Budget), as applicable.

4.4.2 **Current Phase III Protocol and Budget**. The Current Phase III Budget is included in Schedule 4.4.1 (uniQure Development Budget). As may be necessary during the Term, either Party may propose additional Development activities to be included under the Current Phase III Protocol. Upon such a proposal, uniQure will prepare an update to the Current Phase III Protocol (together with a corresponding update to the Current Phase III Budget that contemplates the costs and expenses that uniQure would reasonably be required to incur in connection with the performance of Development activities under such proposed update) and will submit any such update to the JSC to review, discuss, and determine whether to approve in accordance with Article 7 (Governance).

4.4.3 **uniQure Additional Development Plan and Budget**. As may be necessary during the Term, either Party may propose additional Development activities to be performed by uniQure. Upon such a proposal, uniQure will prepare a uniQure Additional Development Plan (together with a corresponding update to the uniQure Additional Development Budget that contemplates the costs and expenses that uniQure would reasonably be required to be incurred in connection with the performance of Development activities under such proposed update) and will submit any such updated plan and budget to the JSC to review, discuss, and determine whether to approve in accordance with Article 7 (Governance).

4.4.4 **Allocation of Costs**. Partner will be responsible for all actual internal costs (at the FTE Rate) and documented out-of-pocket Third Party expenses incurred by or on behalf of uniQure from and after the Effective Date in the performance of any uniQure Development Activities to the extent within the uniQure Development Budget, *plus* Allowable Overruns. Accordingly, after the end of each Financial Quarter during the Term, uniQure will provide to Partner an invoice of the actual internal costs (at the FTE Rate) and documented out-of-pocket Third Party expenses incurred by or on behalf of uniQure in the performance of any uniQure Development Activities during the prior Financial Quarter for which Partner is obligated to reimburse uniQure pursuant to this Section 4.4.4 (Allocation of Costs)

and Partner will pay the undisputed invoiced amounts within [*] after the date of the invoice.

ARTICLE 5
MANUFACTURING

5.1 **Supply Agreement**. Concurrently with the execution of this Agreement, effective as of the Effective Date, the Parties are entering into a supply agreement that sets forth the terms pursuant to which uniQure will supply the Licensed Products to Partner and its Affiliates for Development and Commercialization purposes (the "**Supply Agreement**").

5.2 **Manufacturing Development Plan**. Attached hereto as Schedule 5.2 (Manufacturing Development Plan) is a plan for the activities required to obtain the [*] (the "**Manufacturing Development Plan**"). Either Party may propose updates to the Manufacturing Development Plan to the JSC to review, discuss, and determine whether to approve in accordance with Article 7 (Governance). Once approved by the JSC, each update to the Manufacturing Development Plan will become effective and supersede the previous Manufacturing Development Plan as of the date of such approval or at such other time as decided by the JSC. Notwithstanding any provision to the contrary set forth in this Agreement or in the Supply Agreement, prior to the Manufacturing Responsibility Cutover Date, uniQure will have the right, without seeking JSC approval, to make day-to-day ordinary course operational decisions with respect to the implementation of the Manufacturing Development Plan that do not require an update to the Manufacturing Development Plan in furtherance of the objectives set forth therein. Except (a) as set forth in Section 3.2.3 (Reimbursement of Costs and Expenses) with respect to uniQure's preparation of the Manufacturing portions to be included in the Initial U.S. MAA and Initial E.U. MAA (which activities are further described in "Stage 6" of the Manufacturing Development Plan) and (b) as set forth in Section 7.5.2(b)(ii) (Partner Decisions), Partner will not be responsible or liable for any costs or expenses (internal or Third Party) incurred by uniQure in connection with the performance of activities under the Manufacturing Development Plan. In accordance with the provisions of Section 3.2.3 (Reimbursement of Costs and Expenses) and Section 7.5.2(b)(ii) (Partner Decisions), uniQure may invoice Partner for a Manufacturing Development Plan Required Change Amount, and Partner will pay the undisputed invoiced amounts within [*] after the date of the invoice.

5.3 **Manufacturing Responsibility**.

5.3.1 **uniQure Responsibility**. Subject to Section 5.3.2 (Transfer of Manufacturing Responsibility), during the Term uniQure will use Commercially Reasonable Efforts to supply to Partner and its Affiliates the Licensed Products for Development or Commercialization in accordance with this Agreement and the Supply Agreement and to perform the activities allocated to uniQure under the Manufacturing Development Plan. During the Term, uniQure will not engage any CMO to Manufacture Licensed Products without Partner's prior written consent, not to be unreasonably withheld. If the [*] is achieved at uniQure's Lexington, MA site, then until the Manufacturing Responsibility Cutover Date, uniQure will be responsible for performing all post-marketing commitments and addressing all

comments, required or made by the FDA or EMA with respect thereto, at its sole cost and expense.

5.3.2 **Transfer of Manufacturing Responsibility**. Subject to the last sentence in Section 5.4 (Transfer of Manufacturing Know-How), Partner will have the right to assume responsibility for Manufacturing the Licensed Products for use in the Field in the Territory pursuant to the Manufacturing Responsibility Transfer Plan, which right it may exercise at any time during the Term by providing written notice to uniQure (the date of such notice, the "**Manufacturing Responsibility Transfer Notice Date**"). In such notice, Partner will (a) elect to Manufacture all or part of Partner's requirements for the Licensed Products (i) through the CMO(s) then engaged by uniQure, in which case uniQure will transfer or assign to Partner all applicable agreements between uniQure and such CMO (if and to the extent that such agreement is transferrable or assignable and relates solely to the Licensed Products) or Partner may enter into its own direct agreement with such CMO, (ii) itself, in which case uniQure will transfer the uniQure Manufacturing Know-How to Partner in accordance with Section 5.4 (Transfer of Manufacturing Know-How), or (iii) through a CMO designated by Partner (subject to approval of such CMO by uniQure if required by and in accordance with Section 2.2.2 (Right to Subcontract)), in which case uniQure will transfer the uniQure Manufacturing Know-How to such CMO in accordance with Section 5.4 (Transfer of Manufacturing Know-How), and (b) give uniQure the notice required by Section 14.2 of the Supply Agreement relating to either the termination of the Supply Agreement or the request to have uniQure serve as a second source of Manufacture of the Licensed Products in accordance therewith.

5.3.3 **Manufacturing Responsibility Transfer Plan**. Following the Manufacturing Responsibility Transfer Notice Date, upon Partner's request, the Parties will collaborate reasonably with each other through the JSC to prepare, as soon as reasonably practicable, a written plan for the transfer of uniQure Manufacturing Know-How to Partner or its designated CMO that is not already Manufacturing the Licensed Product, in accordance with the timeline to be set forth therein (the date set forth in such plan for completion of such transfer and assumption of Manufacturing responsibility, the "**Manufacturing Responsibility Cutover Date**"), which plan the JSC may agree to update in accordance with Article 7 (Governance) from time to time (as updated from time to time, the "**Manufacturing Responsibility Transfer Plan**").

5.4 **Transfer of Manufacturing Know-How**. To facilitate Partner's assumption of Manufacturing responsibility in accordance with Section 5.3.2 (Transfer of Manufacturing Responsibility), uniQure will (a) transfer true and complete copies of the uniQure Manufacturing Know-How to Partner or its designated CMO(s) in accordance with the Manufacturing Responsibility Transfer Plan, (b) upon Partner's reasonable request, make available to Partner or its designated CMO(s) a reasonable number of uniQure's technical personnel with appropriate skill and experience at times to be agreed by the Parties, and (c) use Commercially Reasonable Efforts to carry out its obligations under the Manufacturing Responsibility Transfer Plan. Partner will be responsible for all actual

internal costs (at the FTE Rate) and documented out-of-pocket Third Party expenses incurred by uniQure in connection with such transfer of Know-How. Accordingly, uniQure may invoice Partner for such costs and expenses, and Partner will pay the undisputed invoiced amounts within [*] after the date of the invoice. Following completion of uniQure's transfer of Manufacturing responsibility in accordance with Section 5.3.2 (Transfer of Manufacturing Responsibility) and transfer of uniQure Manufacturing Know-How in accordance with the Manufacturing Responsibility Transfer Plan, unless uniQure has agreed to serve as a second source of Manufacture of the Licensed Products in accordance with the terms of the Supply Agreement, uniQure will have no further responsibility for the Manufacture of the Licensed Products. uniQure will be obligated to transfer the uniQure Manufacturing Know-How to Partner or its designated CMO(s) and perform the obligations under the Manufacturing Responsibility Transfer Plan for only one successful transfer; *provided* that, (i) in the event of a transfer to Partner or its designated CMO(s) as a result of a Supply Failure (as defined in the Supply Agreement) pursuant to Section 5.9.2 of the Supply Agreement and thereafter Partner resumes purchase of Licensed Products from uniQure pursuant to Section 5.9.3 of the Supply Agreement (other than as a second source manufacturer in accordance with the proviso therein), then such transfer will not be deemed to be the "one successful transfer" and uniQure will be required to conduct a further successful transfer in accordance with the terms of this Section 5.4 (Transfer of Manufacturing Technology), or (ii) in the event of a transfer to Partner or its designated CMO(s) as a result of a Supply Failure (as defined in the Supply Agreement) pursuant to Section 5.9.2 of the Supply Agreement and thereafter Partner does not resume purchase of Licensed Products from uniQure pursuant to Section 5.9.3 of the Supply Agreement (other than as a second source manufacturer in accordance with the proviso therein), such transfer will be deemed to be the "one successful transfer" and uniQure will not be required to conduct a further successful transfer in accordance with the terms of this Section 5.4 (Transfer of Manufacturing Technology), *provided* that, uniQure will reasonably assist Partner with one additional successful transfer of uniQure Manufacturing Know-How to Partner or its designated CMO thereafter, with such reasonable assistance to be provided at Partner's cost and expense and otherwise in accordance with the terms of this Section 5.4 (Transfer of Manufacturing Know-How).

ARTICLE 6
MEDICAL AFFAIRS AND COMMERCIALIZATION

6.1 **Medical Affairs Responsibilities**. Partner, at its sole cost and expense, will be solely responsible for all Medical Affairs activities for the Licensed Products in the Field in the Territory.

6.2 **Commercialization Responsibilities**. Partner, at its sole cost and expense, will be solely responsible for all Commercialization activities for the Licensed Products in the Field in the Territory. Partner will conduct all Commercialization of each Licensed Product in the Field in the Territory in accordance with the Commercialization Plan for such Licensed Product and subject to the terms of this Agreement and any other written agreement between the Parties with respect to the subject matter set forth herein.

6.3 **Commercialization Diligence**. Following receipt of Regulatory Approval of a Licensed Product in a Major Country, Partner will use Commercially Reasonable Efforts to obtain Reimbursement Approval for such Licensed Product in such Major Country (to the extent it is legally required in such Major Country), and following receipt of Regulatory Approval and (if so required) Reimbursement Approval for a Licensed Product in a Major Country, Partner will use Commercially Reasonable Efforts to Commercialize such Licensed Product in the Field in such Major Country.

6.4 **Partner Commercialization Plan**. No later than [*] after the Effective Date, Partner will develop an initial draft of the written plan for Commercialization of the Licensed Products in the Field in the Territory (as updated from time to time in accordance with this Section 6.4 (Partner Commercialization Plan) and Section 7.2 (Joint Steering Committee), the "**Partner Commercialization Plan**") and provide such initial draft to the JSC to review and discuss. The Partner Commercialization Plan must include in reasonable detail: [*]. Partner will prepare an update to each Partner Commercialization Plan at least annually per Financial Year and will submit each such updated Partner Commercialization Plan to the JSC to review and discuss.

6.5 **Partner Commercialization Reporting**. At least [*] in advance of each JSC meeting, Partner will provide the JSC with a written report detailing Partner's Commercialization activities by Financial Quarter with respect to the Licensed Products, which report will identify all Third Parties performing material Commercialization activities, the key Commercialization activities to be undertaken in the upcoming [*] throughout the Territory, and forecasted Net Sales throughout the Territory for the upcoming [*].

6.6 **Advertising and Promotional Materials**. Partner will be responsible for developing and maintaining all Promotional Materials, which will be consistent and compliant with Applicable Laws, the terms of all applicable Regulatory Approvals, the applicable guidelines from Regulatory Authorities, and Partner's internal medical compliance policy.

6.7 **Product Trademarks**. Partner will own all Product Marks used in connection with the Commercialization of any Licensed Product in the Territory, and have sole responsibility, at its own expense, for all matters relating to the selection and use of such Product Marks, including the selection, filing, prosecution, maintenance, defense, and enforcement thereof. To the extent permitted by Applicable Law and consistent with local industry standard, Partner will use reasonable efforts to include the words "*Developed by uniQure*" in relevant scientific, medical, and other Licensed Product-related communications, or such other similar or otherwise customary text provided by Partner and reasonably acceptable to uniQure.

ARTICLE 7
GOVERNANCE

7.1 **Alliance Managers**. Each Party will appoint an individual to act as its alliance manager under this Agreement as soon as practicable after the Effective Date (each an "**Alliance Manager**"). The Alliance Managers will: (a) serve as the primary points of contact between the Parties for the purpose of providing the other Party with information on the

progress of a Party's activities under this Agreement; (b) be responsible for facilitating the flow of information and otherwise promoting communication, coordination, and collaboration between the Parties; (c) facilitate the prompt resolution of any disputes; and (d) attend JSC meetings, in each case, as a non-voting member. An Alliance Manager may also bring any matter to the attention of the JSC if such Alliance Manager reasonably believes that such matter warrants such attention. Each Party will use reasonable efforts to keep an appropriate level of continuity but may replace its Alliance Manager at any time upon written notice to the other Party.

7.2 Joint Steering Committee.

7.2.1 **Formation and Purpose of JSC**. No later than [*] after the Effective Date, the Parties will establish a joint steering committee (the "**JSC**") to monitor and coordinate the Development and Manufacture of the Licensed Products in the Field in the Territory and to provide a forum for the Parties to discuss and share information regarding the performance of Medical Affairs and Commercialization of the Licensed Products in the Field in the Territory. The JSC will be composed of an equal number of representatives from each Party and a minimum of three representatives of each Party and who have the appropriate and direct knowledge and expertise and requisite decision-making authority. Each Party may replace any of its representatives on the JSC and appoint a person to fill the vacancy arising from each such replacement. A Party that replaces a representative will notify the other Party at least [*] prior to the next scheduled meeting of the JSC. Both Parties will use reasonable efforts to keep an appropriate level of continuity in representation. Representatives may be represented at any meeting by another appropriately knowledgeable person designated by the absent representative. uniQure will designate one of its JSC representatives as one of the co-chairpersons of the JSC and Partner will designate one of its representatives as the other co-chairperson of the JSC. Each Party's representatives on the JSC will inform and coordinate within their respective organization to enable each Party to fulfill its obligations as agreed upon between the Parties under this Agreement, including within the timeframes set forth hereunder.

7.2.2 **JSC Roles and Responsibilities**. The responsibilities of the JSC will be to:

(a) provide a forum for the discussion of the Parties' activities under this Agreement;

(b) review and discuss the Development, Manufacturing, and CMC activities, performance of Medical Affairs, and Commercialization of Licensed Products in the Field in the Territory;

(c) ensure the Assigned Regulatory Materials and each Party's roles and responsibilities therefor, as described in Section 3.5.1 (Regulatory Transfer), are reflected in operation between uniQure and Partner;

(d) receive the Partner Development Plan and any updates thereto, as described

in Section 4.2 (Partner Development Plan);

(e) receive the Development reports, sublicense compliance reports and invention disclosure reports provided by each Party, as described in Section 2.2.5 (Reports of Sublicensees and Subcontractors), Section 4.3 (Development Reporting) and Section 12.1.2 (Disclosure);

(f) review, discuss, and determine whether to approve the plan for the performance of any uniQure Additional Development Activities and, if so approved, the uniQure Additional Development Plan and uniQure Additional Development Budget, as described in Section 4.1.3 (uniQure Additional Development Activities);

(g) review, discuss, and determine whether to approve any update to the Current Phase III Budget, as described in Section 4.4.1 (Current Phase III Protocol);

(h) review, discuss, and determine whether to approve any update to the uniQure Additional Development Plan or uniQure Additional Development Budget, in each case, as described in Section 4.4.3 (uniQure Additional Development Plan and Budget);

(i) oversee the implementation of, and the coordination between the Parties of activities to be performed under, the Supply Agreement, the SDE Agreements, and any other written agreement between the Parties with respect to the Licensed Products in the Field in the Territory;

(j) review, discuss, and determine whether to approve all material updates to the Manufacturing Development Plan, as described in Section 5.2 (Manufacturing Development Plan);

(k) review, discuss, and determine whether to approve all updates to the Manufacturing Responsibility Transfer Plan, as described in Section 5.3.3 (Manufacturing Responsibility Transfer Plan);

(l) review and discuss the Partner Commercialization Plan and any updates thereto, as described in Section 6.4 (Partner Commercialization Plans);

(m) review and discuss the Commercialization reports provided by Partner, as described in Section 6.5 (Partner Commercialization Reporting);

(n) review, discuss, and resolve any disputed change to the Product Specifications requested in accordance with Section 4.1.1 of the Supply Agreement and that has been referred to the JSC in accordance with Section 4.1.3 of the Supply Agreement;

(o) review, discuss, and resolve any disputed change to the Manufacturing Process requested in accordance with Section 4.1.2 of the Supply Agreement and that has been referred to the JSC in accordance with Section

4.1.3 of the Supply Agreement;

(p) oversee the activities of the Parties' Project Managers and the Project Team in carrying out the Manufacturing Development Plan, as described in Section 7.6 (Project Management);

(q) establish and dissolve any subcommittee or working group to discuss specific matters under this Agreement; and

(r) perform such other functions as expressly set forth in this Agreement or allocated to the JSC by the Parties' written agreement.

7.2.3 **Meeting Agendas**. Each Party will disclose to the other Party the proposed agenda items along with appropriate information at least [*] in advance of each meeting of the JSC; *provided* that under exigent circumstances requiring JSC input, a Party may provide its agenda items to the other Party within a shorter period of time in advance of a meeting, or may propose that there not be a specific agenda for a particular meeting, so long as such other Party consents to such later addition of such agenda items or the absence of a specific agenda for such JSC meeting.

7.2.4 **Meetings**. The JSC will hold meetings at such times as it elects to do so but will meet no less frequently than once per Financial Quarter unless otherwise agreed by the Parties. All meetings will be conducted in English unless otherwise agreed by the Parties. The JSC may meet in person or by means of teleconference, Internet conference, videoconference, or other similar communication method; *provided* that at least one meeting each Financial Year will be conducted in person at a location selected alternatively by uniQure and Partner or such other location as the Parties may agree. Each Party will be responsible for all of its own costs and expenses of participating in any JSC meeting. The Alliance Managers will jointly prepare and circulate minutes for each JSC meeting within [*] after each such meeting and will ensure that such minutes are reviewed and approved by their respective companies within [*] thereafter.

7.3 Non-Member Attendance. Each Party may from time to time invite a reasonable number of participants, in addition to its representatives (which may include legal counsel), to attend a meeting of the JSC (in a non-voting capacity), if such participants have expertise that is relevant to the planned agenda for such JSC meeting; *provided* that if either Party intends to have any Third Party (including any consultant) attend such a meeting, then such Party will provide prior written notice to the other Party reasonably in advance of such meeting and will ensure that such Third Party is bound by obligations of confidentiality and non-use at least as stringent as those set forth in Article 9 (Confidentiality; Publication). Notwithstanding any provision to the contrary set forth in this Agreement, if the other Party objects in good faith in writing to the participation of such Third Party in such meeting due to a *bona fide* concern regarding competitively sensitive information that is reasonably likely to be discussed at such meeting *(i.e.*, a consultant that also provides services to a Third Party with a Competitive Product), then such Third Party will not be permitted to

participate in such meeting (or the portion thereof during which such competitively sensitive information is reasonably likely to be discussed).

7.4 Decision-Making.

7.4.1 **General Process**. The JSC, the Project Team, and any Committee established pursuant to this Agreement will only have the powers expressly assigned to it in this Article 7 (Governance) and elsewhere in this Agreement and will not have the authority to: (a) modify or amend the terms of this Agreement; or (b) waive either Party's compliance with the terms of this Agreement. All decisions of the JSC will be made by unanimous vote, with each Party's representatives having collectively one vote (*i.e.*, one vote per Party). No action taken at any meeting of the JSC will be effective unless there is a quorum present at all times at such meeting, and at all such meetings, a quorum will be reached if two voting representatives of each Party are present or participating in such meeting. Except as otherwise expressly set forth in this Agreement, the phrase "determine," "designate," "confirm," "agree," "approve," or "determine whether to approve" by the JSC and similar phrases used in this Agreement will mean approval in accordance with this Section 7.4 (Decision-Making), including the escalation and tie-breaking provisions herein. For the avoidance of doubt, matters that are specified in Section 7.2.2 (JSC Roles and Responsibilities) to be reviewed and discussed (as opposed to reviewed, discussed, and determined) do not require any agreement or decision by either Party and are not subject to the voting and decision-making procedures set forth in this Section 7.4 (Decision-Making) or in Section 7.5 (Resolution of JSC Disputes).

7.4.2 **Decisions of the JSC**. The JSC will use good faith efforts, in compliance with this Section 7.4.2 (Decisions of the JSC), to promptly resolve any such matter for which it has authority. If, after the use of good faith efforts, the JSC is unable to resolve any matter that is within the scope of the JSC's authority or any other disagreement between the Parties that may be referred to the JSC, in each case, within a period of [*] after the applicable meeting of the JSC at which the JSC is unable to reach a resolution, then a Party may refer such matter to the Party's respective Executive Officer for resolution in accordance with Section 7.5.1 (Referral to Executive Officers).

7.5 Resolution of JSC Disputes.

7.5.1 **Referral to Executive Officers**. If a Party makes an election under Section 7.4.2 (Decisions of the JSC) to refer a matter on which the JSC cannot reach a consensus decision for resolution by the Executive Officers, which election must be made no later than [*] after the applicable meeting of the JSC at which the JSC is unable to reach a consensus decision, then the Parties will each submit in writing the respective positions of the Parties to the Executive Officers. The Executive Officers will use good faith efforts to resolve any such matter so referred to them as soon as practicable, and any final decision that the Executive Officers agree to in writing will be conclusive and binding on the Parties.

7.5.2 **Final Decision-Making Authority**. If the Executive Officers are unable to reach agreement on any such matter referred to them within [*] after such matter is so referred (or such longer period as the Executive Officers may agree upon), then:

(a) **No Change; Status Quo**. Neither Party will have final decision-making authority over (i) any matter reasonably likely to give rise to any safety concerns with respect to the Licensed Product, (ii) the conduct of any uniQure Additional Development Activities, (iii) any update with respect to uniQure's activities under the Current Phase III Protocol or Current Phase III Budget, or (iv) the initial uniQure Additional Development Plan or the uniQure Additional Development Budget or any update thereto; *provided* that with respect to each of clauses (ii), (iii), and (iv) above, if a Regulatory Authority requires or requests a change to uniQure Development Activities (including under the Current Phase III Protocol), then Partner will have final decision-making authority in respect of, and will have the right to require such change, if Partner agrees to a corresponding increase to the uniQure Development Budget if such change is made.

(b) **Partner Decisions**. Except for those matters listed in Section 7.5.2(a) (No Change; Status Quo), Partner will have final decision-making authority with respect to (i) all matters related to the Exploitation of the Licensed Products in the Field in the Territory, *provided* that, with respect to any proposed change to Partner's activities under the Current Phase III Protocol, Partner will consider in good faith all reasonable comments of uniQure with respect thereto prior to any exercise of Partner's final decision-making authority over such change, (ii) any changes to the Manufacturing Development Plan (or any further change thereto), *provided* that, if any such change is requested or required by Partner (other than a change that is necessitated by a requirement of a Regulatory Authority [*]) and such change would increase uniQure's expenditures in connection with the performance of the Manufacturing Development Plan or the Manufacturing of the Licensed Products, then Partner may not require such change unless Partner agrees to bear and be liable for the amount of such increase (a "**Manufacturing Development Plan Required Change Amount**"), (iii) the Manufacturing Responsibility Transfer Plan (or any update thereto), subject to Partner's obligations under Section 5.4 (Transfer of Manufacturing Know-How), and (iv) any changes to the Product Specifications (or any update thereto) or to the Manufacturing Process (or any update thereto), *provided* that Partner may not decline to implement any requested change (of either Party) to the Product Specifications or the Manufacturing Process that is required or reasonably anticipated to be required by a Regulatory Authority.

7.5.3 **Limitations on Decision-Making**. Notwithstanding any provision to the contrary set forth in this Agreement, without the other Party's prior written consent, no decision of the JSC or a Party's Executive Officer (in the exercise of a Party's final decision-making authority on any such matters), in each case, may (a) result in a material increase in the other Party's obligations, costs, or expenses under this

Agreement, unless, in each case, such actions are required by a Regulatory Authority or are reasonably necessary for each Party to comply with Applicable Law as the Territory Sponsor or as the owner and holder of any Regulatory Submission, Regulatory Approval, or Reimbursement Approval, as applicable, for the Licensed Product, (b) take or decline to take any action that would be reasonably likely to result in a violation of any Applicable Law, the requirements of any Regulatory Authority, or any agreement with any Third Party (including any agreement pursuant to which uniQure Controls any uniQure Technology) or would be reasonably likely to result in the infringement or misappropriation of intellectual property rights of any Third Party, or (c) conflict with this Agreement, the Supply Agreement, any SDE Agreement, or any other agreement between the Parties related to the subject matter set forth herein.

7.6 **Project Management**. Promptly after the Effective Date, each Party will designate such of their respective employees to form a subcommittee of the JSC to direct the activities to be carried out under the Manufacturing Development Plan ("**Project Team**"). Each Party will also designate one of its employees to act as its project manager (each, a "**Project Manager**"), who will be primarily responsible for day-to-day interactions with the other Party concerning activities performed under the Manufacturing Development Plan and for communicating all instructions and information concerning such activities performed under the Manufacturing Development Plan to the members of the Project Team. The Project Team and Project Managers will consult periodically during the performance of the activities under the Manufacturing Development Plan, through the provision of detailed reports, face-to-face meetings, telephone conferences, videoconferences, or on-site visits during production at times to be agreed upon by the Project Managers. Each Party may appoint a substitute or replacement Project Manager or member(s) of the Project Team in the in the absence of its original Project Manager or original member(s) of the Project Team by notifying the Party in writing of such substitution or replacement. The Project Managers and Project Team will report to and will be subject to the oversight of the JSC. Any dispute arising within the Project Team or between the Parties' respective Project Managers will be referred to the JSC for resolution in accordance with Section 7.4 (Decision-Making). For clarity, the actions of the Project Managers and Project Team will be subject to the final decision-making authority of the JSC as set forth in Section 7.5 (Resolution of JSC Disputes).

7.7 **Committees**. If agreed by the Parties, the JSC may form other committees or working groups as may be necessary or desirable to facilitate the activities under this Agreement (each, a "**Committee**"). A Party may refer any dispute on a matter within a Committee's authority to the JSC for resolution. No such Committees' authority may exceed that specified for the JSC in this Article 7 (Governance).

ARTICLE 8
PAYMENTS

8.1 **Upfront Payment**. No later than [*] after the Effective Date, Partner will pay to uniQure by wire transfer of immediately available funds an upfront payment of $450 million (the "**Upfront Payment**").

8.2 Milestone Payments.

8.2.1 **Regulatory Milestones**. No later than [*] after achievement of the regulatory milestone event set forth below for the Lead Product, Partner will pay to uniQure the corresponding regulatory milestone payment as set forth below (the regulatory milestone events set forth in Table 8.2.1, the "**Regulatory Milestone Events**," and the regulatory milestone payments set forth in Table 8.2.1, the "**Regulatory Milestone Payments**").

Table 8.2.1 – Regulatory Milestones	
Regulatory Milestone Event	***Regulatory Milestone Payment***
[*]	[*]
[*]	[*]
[*]	[*]
[*]	[*]
[*]	[*]
[*]	[*]

8.2.2 **Sales Milestones**. No later than [*] after the end of the first Financial Quarter of a Financial Year in which the total Net Sales of the Licensed Products in the Territory for that Financial Year equals or exceeds one of the thresholds set forth in Table 8.2.2 below, Partner will pay to uniQure the corresponding sales milestone payment set forth in Table 8.2.2 below (the sales milestone events set forth in Table 8.2.2, the "**Sales Milestone Events**" and the sales milestone payments set forth in Table 8.2.2, the "**Sales Milestone Payments**"). For the avoidance of doubt, each Sales Milestone Event may occur only once and the related Sales Milestone Payment may be paid only once.

Table 8.2.2 – Sales Milestones	
Sales Milestone Event	***Sales Milestone Payment***
[*]	[*]
[*]	[*]
[*]	[*]
[*]	[*]
[*]	[*]

8.2.3 **Notification of Milestone Events**. The Party achieving any Regulatory Milestone Event or Sales Milestone Event (each, a "**Milestone Event**") will promptly notify the other Party in writing of, but in no event later than (a) [*] after the achievement of such Regulatory Milestone Event or (b) [*] after the end of the relevant Financial Quarter in which such Sales Milestone Event was achieved. However, in no event will a failure by either Party to deliver such notice of achievement of a Milestone Event relieve Partner of its obligation to pay uniQure the corresponding Regulatory

Milestone Payment or Sales Milestone Payment (each, a "**Milestone Payment**") for achievement of the applicable Milestone Event.

8.2.4 **Payment Adjustment for** [*]. If, at any time, based on communication received from an applicable Regulatory Authority in the United States or Europe, [*], then, upon written notice by Partner to uniQure of such determination setting forth in reasonable detail Partner's rationale for such determination, the Parties in good faith will renegotiate (a) any Milestone Payments for Milestone Events not yet achieved and (b) the Royalty Rates as promptly as reasonably practicable thereafter in light of such additional required Development. If the Parties fail to agree upon the renegotiated Milestone Payments and Royalty Rates within [*] after delivery of such notice, such dispute will be resolved in accordance with the specified expedited arbitration procedures set forth in Section 14.7 (Specified Expedited Arbitrations) and, for the avoidance of doubt, the pre-arbitration negotiation procedure set forth in Section 14.1 (Pre-Arbitration Negotiation) will not apply.

8.2.5 **Payment Condition and Adjustments for** [*] **Milestone**.

(a) If, (i) prior to completion by uniQure of all of its activities under the Manufacturing Development Plan, Partner has assumed Manufacturing responsibility (whether by itself or through any of its Affiliates or designated Subcontractors) for the Licensed Products for use in the Field in the Territory pursuant to Section 5.3.2 (Transfer of Manufacturing Responsibility), including responsibility for the [*], and (ii) as of the Manufacturing Responsibility Transfer Notice Date a CRE Default (as determined pursuant to Section 13.2.2(b) (CRE Defaults)) of uniQure's obligation to perform the activities under the Manufacturing Development Plan in accordance with Section 5.3.1 (uniQure Responsibility) existed (whether determined prior to, on or after the Manufacturing Responsibility Transfer Notice Date), then the Regulatory Milestone Payment for the [*] shall not be payable.

(b) If, (i) prior to completion by uniQure of all of its activities under the Manufacturing Development Plan, Partner has assumed Manufacturing responsibility (whether by itself or through any of its Affiliates or designated Subcontractors) for the Licensed Products for use in the Field in the Territory pursuant to Section 5.3.2 (Transfer of Manufacturing Responsibility), including responsibility for the [*], and (ii) as of the Manufacturing Responsibility Transfer Notice Date a CRE Default (as determined pursuant to Section 13.2.2(b) (CRE Defaults)) of uniQure's obligation to perform the activities under the Manufacturing Development Plan in accordance with Section 5.3.1 (uniQure Responsibility) did not exist (whether determined prior to, on or after the Manufacturing Responsibility Transfer Notice Date), then the Parties in good faith will negotiate an equitable reduction of the Regulatory Milestone Payment payable to uniQure upon achievement of the [*] based on each Party's *pro rata* contributions to the achievement of such milestone event (*e.g.*, if Partner or

its Affiliate or designated Subcontractor contributed [*]% of the activities required to obtain the [*], then such Regulatory Milestone Payment would be reduced by [*]%), and Partner will pay to uniQure only that agreed percentage of such Regulatory Milestone Payment attributable to uniQure's contribution to achievement of the [*]. If the Parties fail to agree upon such apportionment, then such dispute will be resolved in accordance with the specified expedited arbitration procedures set forth in Section 14.7 (Specified Expedited Arbitrations) and, for the avoidance of doubt, the pre-arbitration negotiation procedure set forth in Section 14.1 (Pre-Arbitration Negotiation) will not apply.

(c) If (i) uniQure performs and completes its activities under the Manufacturing Development Plan in accordance with Section 5.3.1 (uniQure Responsibility) and the [*] is achieved, but (ii) (A) such [*] has not been achieved in the United States prior to the [*], then the Regulatory Milestone Payment payable to uniQure upon achievement of the [*] will be reduced by an amount equal to the difference between (x) the aggregate amount actually paid or payable by Partner to uniQure [*] and (y) the aggregate amount that would have been paid or payable by Partner to uniQure [*], or (B) such [*] has not been achieved in the E.U. prior to the [*] of the receipt of Regulatory Approval of the Initial E.U. MAA, then the Regulatory Milestone Payment payable to uniQure upon achievement of the [*] will be reduced by an amount equal to the difference between (I) the aggregate amount actually paid or payable by Partner to uniQure [*] and (II) the aggregate amount that would have been paid or payable by Partner to uniQure [*].

8.3 Royalties.

8.3.1 **Royalty Payments**. Partner will pay to uniQure, on a country-by-country basis, royalties during the Term at the applicable royalty rates set forth in Table 8.3.1 (the "**Royalty Rates**"), on an incremental basis, on the aggregate, worldwide Net Sales of any Licensed Product by Partner and its Affiliates and Sublicensees in the Territory in the applicable Financial Year (the "**Royalties**") until the latest to occur of (a) [*] after First Commercial Sale of a Licensed Product in such country, (b) the expiration of the last Valid Claim in the uniQure Royalty Patent Rights that Cover such Licensed Product or the making, using, or selling thereof in such country and (c) [*] (the "**Royalty Term**"). [*]

Table 8.3.1 – Royalty Rates

Worldwide Annual Net Sales	***Royalty Rate***
The portion that is ≤$[*]	[*]%
The portion that is >$[*] and ≤$[*]	[*]%
The portion that is >$[*]	[*]%

8.3.2 **Royalty Reductions**.

(a) **Patent Expiration Step-Down**. Subject to Section 8.3.2(d) (Royalty Reductions Floor), on a Licensed Product-by-Licensed Product and country-by-country basis, the royalty rates payable by Partner with respect to the Net Sales of a Licensed Product in a country in the Territory will be reduced by [*]% during each Financial Quarter following the expiration or invalidation of the last Valid Claim within the uniQure Royalty Patent Rights that Covers such Licensed Product or the making, using, or selling thereof in such country; *provided* that [*].

(b) **Loss of Market Exclusivity**. If, on a Licensed Product-by-Licensed Product, Financial Quarter-by-Financial Quarter, and country-by-country basis, during the Royalty Term of a Licensed Product in a country, (i) there is a Loss of Market Exclusivity with respect to such Licensed Product or (ii) [*], then, subject to Section 8.3.2(d) (Royalty Reductions Floor), the royalty rates payable by Partner pursuant to Section 8.3.1 (Royalty Payments) with respect to the Net Sales of such Licensed Product in such country in such Financial Quarter will be reduced by [*]%.

(c) **Right to Offset**. Subject to Section 8.3.2(d) (Royalty Reductions Floor), on a Licensed Product-by-Licensed Product and country-by-country basis, Partner may credit [*]% of any Third Party Royalty Payments with respect to a Licensed Product in a country in the Territory in a Financial Quarter against the Royalties due and payable by Partner to uniQure on the Net Sales for such Licensed Product in such country in such Financial Quarter.

(d) **Royalty Reductions Floor**. On a Licensed Product-by-Licensed Product, Financial Quarter-by-Financial Quarter, and country-by-country basis, in no event will the Royalties due to uniQure for a Licensed Product in a country in the Territory in any given Financial Quarter during the Royalty Term for such Licensed Product in such country be reduced by more than [*]% of the amount that otherwise would have been due and payable to uniQure in such Financial Quarter for such Licensed Product in such country but for the reductions set forth in this Section 8.3.2 (Royalty Reductions). Partner may carry forward any such reductions permitted under this Section 8.3.2 (Royalty Reductions) that are incurred or accrued in a Financial Quarter but are not applied against Royalties due to uniQure in such Financial Quarter as a result of the foregoing floor and apply such amounts against Royalties due to uniQure in any subsequent Financial Quarter (subject to the minimum floor set forth in this Section 8.3.2(d) (Royalty Reductions Floor)) until the amount of such reduction has been fully applied against Royalties due to uniQure.

8.3.3 **Royalty Payments and Reports**.

(a) **Flash Reports**. Within [*] after the end of each Financial Quarter during the Term, Partner will provide to uniQure flash reports that will set forth (i) for the first and second month of such Financial Quarter: (A) the actual

gross sales of each Licensed Product sold by Partner or its Affiliates or Sublicensees in the Territory in such months; and (B) the actual total aggregate Net Sales of each Licensed Product sold by Partner or its Affiliates or Sublicensees in the Territory in such months; and (ii) for the third month of such Financial Quarter, Partner's good faith estimate of the amounts set forth in the foregoing clauses (i)(A) and (i)(B) of this Section 8.3.3(a) (Flash Reports) (the "**Flash Report**").

(b) **Royalty Reports**. In addition to the Flash Reports to be provided in accordance with Section 8.3.3(a) (Flash Reports), within [*] after the end of each Financial Quarter during the Term, Partner will provide to uniQure a written report (each, a "**Royalty Report**") setting forth in reasonable detail (i) the gross sales of each Licensed Product sold by Partner or its Affiliate or Sublicensee in the Territory in such Financial Quarter; (ii) the aggregate Net Sales of each Licensed Product sold by Partner or its Affiliates or Sublicensees in the Territory in such Financial Quarter; (iii) all deductions and reductions used to determine the Net Sales of each Licensed Product for such Financial Quarter and the Royalties payable with respect to each Licensed Product for such Financial Quarter, including any reductions taken pursuant to Section 8.3.2 (Royalty Reduction); (iv) the exchange rates used to calculate the Royalties payable in U.S. Dollars; (v) any withholding taxes required to be made from such Royalties; and (vi) the quantity and description of each Licensed Product sold by Partner or its Affiliate or Sublicensee in the Territory during such Financial Quarter comprising such Net Sales, including detailed sales reports for each Licensed Product for each month of the Financial Quarter in each country in the Territory. The amounts, calculations, and information set forth in the foregoing clauses (i) through (vi) will be broken down on a monthly basis for each country in the Territory. The Parties will seek to resolve any questions or issues related to a Royalty Report within [*] following receipt by uniQure of each Royalty Report.

(c) **Royalty Payments**. The information contained in each Flash Report and Royalty Report will be considered Confidential Information of Partner. Within [*] after the end of each Financial Quarter, Partner will make the Royalty payment due hereunder for the Financial Quarter covered by the applicable Royalty Report.

8.4 **Development Payment**. Partner will partially fund uniQure's Development expenses between the Execution Date and the earlier of the Effective Date or the date this Agreement is terminated pursuant to Section 15.5 (Outside Date) by paying uniQure a one-time, aggregate payment in an amount equal [*] (such amount, the "**Development Payment**"). Partner will pay uniQure the Development Payment no later than [*] after the earlier of the Effective Date or the date this Agreement is terminated pursuant to Section 15.5 (Outside Date). The Development Payment will be irrevocable, non-refundable, and non-creditable against any other payment due to uniQure pursuant to this Agreement (subject only to the terms of Section 15.5 (Outside Date)).

8.5 **Other Amounts Payable**. With respect to any amounts owed under this Agreement by one Party to the other for which no other invoicing and payment procedure is specified hereunder, within [*] after the end of each Financial Quarter, each Party will provide an invoice, together with reasonable supporting documentation, to the other Party for such amounts owed in respect of such Financial Quarter. The owing Party will pay any undisputed amounts within [*] after the receipt of the invoice, and any disputed amounts owed by a Party will be paid within [*] after resolution of the dispute.

8.6 **Offset for "Step-In" Exercise**. Partner may offset 100% of any amounts paid by Partner to any Third Party resulting from Partner's cure of any uncured breach by uniQure under any Existing In-License pursuant to Section 2.5(f) (Existing In-Licenses) against any payment(s) then or thereafter due to uniQure under this Agreement.

8.7 **No Refunds**. Except as expressly provided herein (including Section 8.12 (Financial Records and Audits) and Article 11 (Indemnification)), all payments under this Agreement will be irrevocable, non-refundable, and non-creditable (absent manifest calculation error or payment processing error).

8.8 **Accounting Standards**. If a Party changes its general accounting principles from the then-current standard (*e.g.*, from GAAP to IFRS) at any time during the Term, then at least [*] prior to adopting such change in principles, such Party will provide written notice to the other Party of such change.

8.9 **Currency; Exchange Rate**. All amounts expressed in this Agreement are expressed in U.S. Dollars. All payments to be made by Partner to uniQure under this Agreement will be made in U.S. Dollars by electronic funds transfer in immediately available funds to a bank account designated in writing by uniQure. All payments to be made by uniQure to Partner under this Agreement will be made in U.S. Dollars by electronic funds transfer in immediately available funds to a bank account designated in writing by Partner. Conversion of Net Sales recorded in local currencies will be converted to U.S. Dollars at the exchange rate used by CSL Limited in accordance with its Accounting Standards on the last Business Day of the Financial Quarter in which the applicable payment obligation became due and payable.

8.10 **Blocked Payments**. If by reason of Applicable Law in any country or region, it becomes impossible or illegal for a Party to transfer, or have transferred on its behalf, payments owed to the other Party hereunder, then such Party will promptly notify the other Party of the conditions preventing such transfer and such payments will be deposited in local currency in the relevant country or region to the credit of the other Party in a recognized banking institution designated by the other Party or, if none is designated by the other Party within a period of [*], in a recognized banking institution selected by the transferring Party, as the case may be, and identified in a written notice given to the other Party.

8.11 **Late Payments**. Any payments or portions thereof due hereunder that are not paid on the date such payments are due under this Agreement will bear interest at a rate equal to the lesser of: (a) [*] percentage points above the Prime Rate as quoted in *The Wall Street Journal* on the first day of each Financial Quarter in which such payments are overdue; or

(b) the maximum rate permitted by Applicable Law, in each case, calculated on the number of days such payment is delinquent, compounded monthly.

8.12 **Financial Records and Audits**. Each Party will maintain complete and accurate records for at least [*] following the relevant Financial Quarter to which they pertain in sufficient detail to permit the other Party to confirm the accuracy of the amount of costs and expenses incurred in connection with the performance of the uniQure Development Activities, Royalties, and other amounts payable under this Agreement. Upon reasonable prior written notice, such records will be made available during regular business hours for examination by an independent certified public accountant of recognized international standing selected by the examining Party and reasonably acceptable to the other Party for the sole purpose of verifying for the examining Party the accuracy of the financial reports furnished by the other Party (the "**Examined Party**") pursuant to this Agreement or of any payments made, or required to be made, by such Examined Party pursuant to this Agreement; *provided* that such independent accounting firm is subject to customary written obligations of confidentiality and non-use applicable to each Party's Confidential Information. Such audit will not be (a) performed more frequently than once per Financial Year during the Term or once during the [*] period after the expiration or termination of this Agreement, (b) conducted for any Financial Year more than [*] after the end of such year, or (c) repeated for any Financial Year or with respect to the same set of records (unless a material discrepancy with respect to such records is discovered during a subsequent audit). Such accountant will not disclose the Examined Party's Confidential Information to the examining Party or to any Third Party, except to the extent such disclosure is necessary to verify the accuracy of the financial reports furnished by the Examined Party or the amount of payment by the Examined Party under this Agreement. If such accountant concludes either that an additional amount is owed to a Party but unpaid, or that an amount paid by a Party was in excess of the amount owed, then, in either case, the Party that owes the other Party an amount (*i.e.*, the amount owed but unpaid by such Party or the amount that such Party was overpaid) will pay such amount *plus* interest (as set forth in Section 8.11 (Late Payments)) from the original due date or the overpayment date, as applicable) to the other Party within [*] after the delivery by the accountant of the accountant's written report to the Parties so concluding. If each Party owes an amount(s) to the other Party, then the accountant will net such amounts (including the interest that would be payable thereon), such that only the Party that owes the larger aggregate amount to the other Party will be required to make a payment as a result of such audit. The examining Party will bear the full cost of such audit unless such audit reveals that the Examined Party owes the examining Party an amount that is more than [*] percent ([*]%) of the amount actually due for the time period being audited, in which case the Examined Party will reimburse the examining Party for the reasonable audit fees for such examination.

8.13 **Taxes**. If under any Applicable Law or regulation of any country of the Territory withholding of Taxes of any type, levies, or other charges is required with respect to any amounts payable hereunder to a Party, the other Party ("**Withholding Party**") will apply the withholding or deduction as so required and will promptly pay such Tax, levy, or charge to the proper Governmental Authority, and will promptly furnish the Party with proof of such payment. The Withholding Party will have the right to withhold or deduct any such Tax, levy, or charge actually paid from payment due to the Party or be promptly reimbursed

by the Party if no further payments are due to the Party. Any amounts so withheld or deducted from the payment due to the Party pursuant to the relevant law or regulation will be deemed paid to such Party for all purposes of this Agreement. Each Withholding Party agrees to assist the other Party in claiming exemption from (or reduction in) such deductions or withholdings under double taxation or similar agreement or treaty from time-to-time in force and in minimizing the amount required to be so withheld or deducted. Notwithstanding the foregoing, all sums payable by either Party hereunder are stated exclusive of any sales tax, value added tax, or other similar taxes, assessments, and charges imposed by the jurisdiction of the Withholding Party or the payee and any such taxes will be paid by the Withholding Party, except in the case that the paying Party assigns, transfers, or otherwise disposes of some or all of its rights and obligations to any Person and if, as a result of such action, the withholding or deduction of Tax required by Applicable Law with respect to payments under this Agreement is increased (without an offsetting credit that can be used by the other Party in the current tax year), in which case any amount payable to the other Party under this Agreement will be increased by the incremental amount of such withholding or deduction of Tax (determined on a "with or without" basis) (the "**Incremental Withholding Amount**"). If the other Party is subsequently able to claim a credit from the relevant Governmental Authority in an amount that includes all or a part of the Incremental Withholding Amount, then the other Party will notify the Withholding Party of such event and will apply such amount as an offset or rebate against any amounts payable by the Withholding Party to the other Party under this Agreement.

ARTICLE 9
CONFIDENTIALITY; PUBLICATION

9.1 **Duty of Confidence**. Subject to the other provisions of this Article 9 (Confidentiality; Publication):

9.1.1 except to the extent expressly authorized by this Agreement, all Confidential Information of a Party (the "**Disclosing Party**") or its Affiliates will be maintained in confidence and otherwise safeguarded, and not published or otherwise disclosed, by the other Party (the "**Receiving Party**") and its Affiliates for the Term and for [*] thereafter;

9.1.2 the Receiving Party will secure and protect all Confidential Information provided by the Disclosing Party with the same degree of care as the Receiving Party uses for its own similar information, but in no event less than a reasonable degree of care in light of the sensitivity of the Confidential Information in question;

9.1.3 the Receiving Party may only use any Confidential Information of the Disclosing Party for the purposes of performing its obligations or exercising its rights under this Agreement;

9.1.4 a Receiving Party may disclose Confidential Information of the Disclosing Party to: (a) such Receiving Party's Affiliates, licensees, Sublicensees, and Subcontractors; and (b) employees, directors, officers, agents, contractors,

consultants, attorneys, accountants, banks, investors, and advisors of the Receiving Party and its Affiliates, licensees, Sublicensees, and Subcontractors in each case ((a) and (b)), to the extent reasonably necessary for the purposes of, and for those matters undertaken pursuant to, this Agreement or in connection with rendering advice or other services to the Receiving Party; *provided* that such Persons are bound by obligations of confidentiality and non-use with respect to the Disclosing Party's Confidential Information no less stringent than the confidentiality and non-use obligations set forth in this Agreement or otherwise customary in light of the purposes for which such disclosure is being made. Each Party will remain responsible for any failure by its Affiliates, licensees, Sublicensees, and Subcontractors, and its and its Affiliates', licensees', and Sublicensees' respective employees, directors, officers, agents, consultants, attorneys, accountants, banks, investors, advisors, and contractors, in each case, to treat such Confidential Information as required under this Section 9.1 (Duty of Confidence) as if such Affiliates, licensees, Sublicensees, Subcontractors, employees, directors, officers agents, consultants, advisors, attorneys, accountants, banks, investors, and contractors were Parties directly bound to the requirements of this Section 9.1 (Duty of Confidence); and

9.1.5 each Party will promptly notify the other Party if the first Party becomes actually aware of any misuse or unauthorized disclosure of the other Party's Confidential Information and take all necessary actions to remediate and comply with all Applicable Laws regarding same.

9.2 Confidential Information. The uniQure Know-How will be the Confidential Information of uniQure. The Joint Know-How and the terms of this Agreement will be the Confidential Information of each Party. The Partner Know-How will be the Confidential Information of Partner. Except as otherwise expressly permitted by or authorized in accordance with this Agreement, neither Party nor its Affiliates may disclose the existence or the terms of this Agreement.

9.3 Exemptions. Information of a Disclosing Party will not be Confidential Information of such Disclosing Party to the extent that the Receiving Party can demonstrate through reasonable evidence that such information:

9.3.1 is known by the Receiving Party or any of its Affiliates without an obligation of confidentiality to any Person at the time of its receipt from or on behalf of the Disclosing Party or its Affiliates, and not through a prior disclosure by or on behalf of the Disclosing Party or its Affiliates, as documented by the Receiving Party's business records;

9.3.2 is generally available to the public before its receipt from or on behalf of the Disclosing Party;

9.3.3 becomes generally available to the public or otherwise part of the public domain after its disclosure by or on behalf of the Disclosing Party or its Affiliates and other

than through any act or omission of the Receiving Party or any of its Affiliates or other disclosees in breach of this Agreement;

9.3.4 is subsequently disclosed to the Receiving Party or any of its Affiliates without obligation of confidentiality by a Third Party who may rightfully do so and, to the actual knowledge of the Receiving Party or such Affiliate, is not under any obligation of confidentiality to the Disclosing Party or its Affiliates; or

9.3.5 is developed by the Receiving Party or any of its Affiliates independently and without use of or reference to any Confidential Information received from or on behalf of the Disclosing Party or any of its Affiliates, as documented by the Receiving Party's business records.

9.4 Authorized Disclosures. Notwithstanding the obligations set forth in Section 9.1 (Duty of Confidence), a Party may disclose the other Party's Confidential Information (including the existence and terms of this Agreement) in the following situations:

9.4.1 (a) the Patent Prosecution of uniQure Patent Rights, Joint Patent Rights, or Partner Patent Rights, in each case, as contemplated by this Agreement; or (b) Regulatory Submission and other filings with Governmental Authorities (including Regulatory Authorities), as necessary for the Exploitation of any Licensed Product in accordance with the rights and obligations of the applicable Party under this Agreement;

9.4.2 disclosure of this Agreement, its terms, and the status and results of Exploitation of any Licensed Product to actual or *bona fide* potential investors, acquirors, (sub)licensees, lenders, and other financial or commercial partners (including in connection with any royalty factoring transaction), and their respective attorneys, accountants, banks, investors, and advisors, solely for the purpose of evaluating or carrying out an actual or potential investment, acquisition, (sub)license, debt transaction, or collaboration; *provided* that, in each such case, subject to Applicable Laws and on the condition that such Persons are bound by obligations of confidentiality and non-use at least as stringent as those set forth in this Article 9 (Confidentiality; Publication) or otherwise customary for such type and scope of disclosure; *provided* that any such disclosure is limited to the maximum extent practicable for the particular context in which it is being disclosed;

9.4.3 such disclosure is required to comply with Applicable Law (whether generally or in pursuit of an application for listing of securities), including the United States Securities and Exchange Commission or equivalent foreign agency or regulatory body, or otherwise required by or requested pursuant to any judicial, administrative or other legal process, *provided* that in each such event, as promptly as reasonably practicable and to the extent not prohibited by Applicable Law, such Party will notify the other Party of such required or requested disclosure and provide a draft of the disclosure to the other Party reasonably in advance of such disclosure for the other Party's review and comment. The non-disclosing Party will provide any comments as soon as practicable, and the disclosing Party will consider in good

faith any timely comments provided by the non-disclosing Party; *provided* that the disclosing Party may or may not accept such comments in its sole discretion. Confidential Information that is disclosed in order to comply with Applicable Law or by judicial, administrative or other legal process pursuant to this Section 9.4.3, in each case, will remain otherwise subject to the confidentiality and non-use provisions of this Article 9 (Confidentiality; Publication) with respect to the Party disclosing such Confidential Information, and at the request of the non-disclosing Party, such disclosing Party will take all steps reasonably necessary, including seeking of confidential treatment or a protective order, to ensure the continued confidential treatment of such Confidential Information, and each Party will be responsible for its own legal and other out-of-pocket Third Party costs in connection with any such filing or disclosure pursuant to this Section 9.4.3. If a Party is required or permitted to make a disclosure of the other Party's Confidential Information pursuant to this Section 9.4.3 subject to complying with the other provisions of this Section 9.4.3, a disclosing Party may disclose only that portion of Confidential Information that is legally required to be disclosed as advised by its legal counsel; or

9.4.4 disclosure pursuant to Section 9.6 (Publications) or Section 9.7 (Publicity; Use of Name).

9.5 **Tax Treatment**. Nothing in Section 9.1 (Duty of Confidence) or Section 9.4 (Authorized Disclosures) will limit either Party in any way from disclosing to any Third Party such Party's U.S. or foreign income Tax treatment and the U.S. or foreign income Tax structure of the transactions relating to such Party that are based on or derived from this Agreement, or materials of any kind (including opinions or other Tax analyses) relating to such Tax treatment or Tax structure, except to the extent that nondisclosure of such matters is reasonably necessary in order to comply with applicable securities laws.

9.6 **Publications**. Partner may publicly present or publish any Clinical Trial or Commercialization data, non-clinical or preclinical data, or any associated results, data, or conclusions generated by or on behalf of Partner pursuant to this Agreement (each such proposed presentation or publication, a "**Publication**"), *provided* that such Publication does not contain any Confidential Information of uniQure (without uniQure's prior written consent). Partner will consult reasonably with uniQure with respect to Partner's publication and communication strategy with respect to the Licensed Products in the Field in the Territory. If Partner desires to publicly present or publish a Publication relating in any way to the Manufacture of a Licensed Product or any uniQure Manufacturing Technology, and such Publication (a) is not a Promotional Material and (b) contains substantive information not previously publicly presented or published, then Partner will provide uniQure with a copy of such proposed Publication at least [*] prior to the earlier of its presentation or intended submission for publication (such applicable period, the "**Review Period**"), and Partner agrees that it will not submit or present any such Publication until (i) uniQure has provided written comments during such Review Period on the material in such Publication, or (ii) the applicable Review Period has elapsed without written comments from uniQure, in which case Partner may proceed and the Publication will be considered approved in its entirety. Notwithstanding any provision to contrary set

forth in this Agreement, Partner will (x) delete any Confidential Information of uniQure that uniQure identifies for deletion in uniQure's written comments and (y) delay such Publication for a period of up to an additional [*] after the end of the applicable Review Period to enable uniQure to draft and file one or more patent applications with respect to information of uniQure relating to the Manufacture of a Licensed Product or any uniQure Manufacturing Technology (and not any information of Partner or its Affiliates or Sublicensees) to be made public in such Publication. After a Publication has been made available to the public, each Party may post such Publication or a link to it on its corporate website (or any website managed by such Party in connection with a Clinical Trial for a Licensed Product, as appropriate) or republish it in any other way, without the prior written consent of the other Party, so long as the information in such Publication remains true, correct, and contain the most current information with respect to the subject matters set forth therein.

9.7 Publicity; Use of Names.

9.7.1 **Press Release**. The Parties have agreed on the content of separate press releases announcing this Agreement, each as set forth on Schedule 9.7.1 (Press Releases), to be issued by the applicable Party on such date and time as may be agreed by the Parties. Other than the press releases set forth on Schedule 9.7.1 (Press Releases) and the disclosures permitted by this Section 9.7 (Publicity; Use of Names) and Section 9.4 (Authorized Disclosures), the Parties agree that the portions of any other press release or other public action or announcement relating to this Agreement or the performance hereunder that would disclose information, other than that which is not Confidential Information of a Party and so long as such information remains true, correct, and current, will first be reviewed and approved by both Parties (with such approval not to be unreasonably withheld).

9.7.2 **Use of Names**. Each Party will have the right to use the other Party's name and logo as otherwise set forth in this Agreement and in presentations, its website, collateral materials, and corporate overviews to describe the collaboration relationship, as well as in taglines of press releases issued pursuant to this Section 9.7 (Publicity; Use of Names); *provided* that each Party will use the other Party's corporate name in such manner (a) that the distinctiveness, reputation, and validity of any trademarks and corporate or trade names of such other Party will not be impaired, (b) consistent with best practices used by such other Party for its other collaborators, and (c) in accordance with the other Party's written approval (with such approval not to be unreasonably withheld), and *provided further* that each Party shall provide to the other Party reasonable prior written notice of its intention to use the other Party's corporate name, but once a Party gives its initial approval to such use, its further approval is not required for any subsequent uses that do not materially differ from such approved use. Except as permitted under this Section 9.7 (Publicity; Use of Names) or with the prior express written permission of the other Party, neither Party will use the name, trademark, trade name, or logo of the other Party or its Affiliates or their respective employees in any publicity, promotion, news release, or disclosure relating to this Agreement or its subject matter except as may be required by Applicable Law. Each Party will

use the other Party's corporate name in the form and format provided or otherwise approved by such other Party in all publicity relating to this Agreement, including the initial press release and all subsequent press releases. To the extent permitted by Applicable Law, Partner will use reasonable efforts to include explanatory text such as "*Developed by uniQure*" in all publicity, promotion, news releases, or disclosures relating to the Licensed Product, or such other similar or otherwise customary text.

9.8 **Attorney-Client Privilege**. Neither Party is waiving, nor will be deemed to have waived or diminished, any of its attorney work product protections, attorney-client privileges or similar protections and privileges or the like as a result of disclosing information pursuant to this Agreement, or any of its Confidential Information (including Confidential Information related to pending or threatened litigation) to the Receiving Party, regardless of whether the Disclosing Party has asserted, such privileges and protections. The Parties: (a) share a common legal and commercial interest in such disclosure that is subject to such privileges and protections; (b) are or may become joint defendants in proceedings to which the information covered by such protections and privileges relates; (c) intend that such privileges and protections remain intact should either Party become subject to any actual or threatened proceeding to which the Disclosing Party's Confidential Information covered by such protections and privileges relates; and (d) intend that after the Effective Date both the Receiving Party and the Disclosing Party will have the right to assert such protections and privileges. Notwithstanding any provision to the contrary set forth in this Agreement, nothing in this Section 9.8 (Attorney-Client Privilege) will apply with respect to a Dispute between the Parties (including their respective Affiliates).

9.9 **Personal Information.** Each Party will comply with all Applicable Laws with respect to its collection, processing, use, disclosure, sharing, transfer, storage, and disposal of personal or personally identifiable information in connection with this Agreement. The Parties will fully cooperate in such process and execute all additional agreements that are necessary or desirable to further same.

ARTICLE 10
REPRESENTATIONS, WARRANTIES, AND COVENANTS

10.1 **Representations and Warranties of Each Party**. Each Party represents and warrants to the other Party as of the Execution Date as follows:

10.1.1 It is a corporation or limited company duly organized, validly existing, and, as applicable, in good standing under the laws of the jurisdiction of its organization, and it has the full right, power and authority to enter into this Agreement and to perform its obligations hereunder.

10.1.2 It has not been Debarred/Excluded and no proceeding that could result it in being Debarred/Excluded is pending, and neither it nor any of its Affiliates has used, in any capacity in the performance of obligations relating to the Licensed Product, any employee, subcontractor, consultant, agent, representative, or other Person who has been Debarred/Excluded.

10.1.3 All consents, approval, and authorizations from all Governmental Authorities (other than (a) as of the Execution Date, those required under applicable Antitrust Laws and (b) the Regulatory Approvals and, if applicable, any Reimbursement Approvals contemplated to be obtained pursuant to this Agreement) or other Third Parties (including, with respect to uniQure, under all Existing In-Licenses) required to be obtained by such Party in connection with this Agreement or to perform all of the actions contemplated hereby have been obtained.

10.1.4 This Agreement has been duly executed by it and is legally binding upon it, enforceable in accordance with its terms, and does not conflict with any agreement, instrument or understanding, oral or written, to which it is a party or by which it may be bound, nor (assuming all consents, approvals, and authorizations contemplated in clause (a) of Section 10.1.3 have been obtained) violate any material Applicable Law or regulation of any court, governmental body, or administrative or other agency having jurisdiction over it.

10.1.5 Such Party has sufficient financial wherewithal to (a) perform all of its obligations set forth under this Agreement, and (b) meet all of its obligations that come due in the ordinary course of business.

10.2 Representations and Warranties of uniQure. uniQure represents and warrants to Partner as of the Execution Date as follows:

10.2.1 uniQure and its Affiliates are the sole legal and beneficial owner of all of the uniQure Technology with respect to the Licensed Product that is not licensed to uniQure under the Existing In-Licenses, free and clear of all Encumbrances other than as set forth on Schedule 10.2.1 (Permitted Encumbrances), and have sufficient rights under the uniQure Technology to grant to Partner the licenses set forth in this Agreement, and none of them have granted to any other Person any license or other right under the uniQure Technology with respect to the Licensed Product.

10.2.2 Schedule 10.2.2 (uniQure Patent Rights) sets forth the uniQure Patent Rights that are owned or exclusively licensed by uniQure or any of its Affiliates in the Territory and that exist as of the Execution Date. With respect to any uniQure Patent Right identified on Schedule 10.2.2 (uniQure Patent Rights) as being owned by uniQure or any of its Affiliates, uniQure or such Affiliate owns all rights, title, and interests in and to such uniQure Patent Rights, free and clear of all Encumbrances other than as set forth on Schedule 10.2.1 (Permitted Encumbrances), or any claims or interests of any current or former employee or contractor with respect to the Licensed Product.

10.2.3 All issued patents included in the uniQure Patent Rights are subsisting and unexpired and, to uniQure's Knowledge, valid and enforceable, and all filings and fees required to maintain such issued patents prior to the Execution Date have been timely and satisfactorily made. UniQure has at all times complied with its duty of disclosure or candor to any Governmental Authority that issues patents and has not engaged in any act or omission at any such Governmental Authority that would

support a finding of fraud, inequitable conduct, or similar principles under Applicable Law, in each case, with respect to any patents or applications included in the uniQure Patent Rights.

10.2.4 uniQure has provided Partner with unredacted and otherwise true copies of each Existing In-License entered into by uniQure or its Affiliates and listed on Schedule 2.5 (Existing In-Licenses) as of the Execution Date. The licenses on Schedule 2.5 (Existing In-Licenses) are all of the licenses of intellectual property pursuant to which uniQure or any of its Affiliates Control any uniQure Technology. Neither uniQure nor any of its Affiliates is or is alleged to be in default of any Existing In-License and, to uniQure's Knowledge, no other party to any Existing In-License is in default thereof. The actions contemplated by this Agreement will not violate any Existing In-License or give any other party the right to modify its terms in any manner adverse to Partner with respect to the Licensed Products. All parties to the Existing In-Licenses are, to uniQure's Knowledge, in good standing thereunder, and no such party has issued or received any notice of breach, default, termination, cancellation or non-renewal with respect thereto. uniQure has performed its obligations, if any, under any applicable Existing In-License in order to allow Partner to obtain a direct patent license on substantially the same terms in the event of termination of uniQure's license in the applicable circumstances.

10.2.5 There is no pending or, to uniQure's Knowledge, threatened (in writing) claim, action, litigation, or proceeding, nor has uniQure or its Affiliates received any written notice from any Third Party, asserting or alleging that (a) the Exploitation of the Lead Product in the Field prior to the Execution Date in or for the Territory infringed or misappropriated the intellectual property rights of such Third Party or (b) except as set forth on Schedule 10.2.5 (Third Party Claims), any of the uniQure Technology is invalid or unenforceable.

10.2.6 To uniQure's Knowledge, the Manufacturing, Development and Commercialization of the Licensed Products will not infringe or violate the intellectual property of any Person. For the purposes of this Section 10.2.6, "uniQure's Knowledge" takes into account the contents of uniQure's periodic, ordinary course freedom-to-operate monitoring and analysis, and reasonable belief.

10.2.7 Except to the extent set forth in any Existing In-License, no funds, personnel, equipment, or resources of any Governmental Authority, university, college, research, or other institution were used in connection with the invention or development of any uniQure Technology, and neither uniQure nor any of its Affiliates has any obligation to grant to any Third Party any license or consent under the uniQure Technology in a manner that is inconsistent with the licenses granted to Partner with respect to the Licensed Products hereunder.

10.2.8 There are no legal claims, judgments, or settlements against or owed by uniQure or any of its Affiliates, or pending or, to uniQure's Knowledge, threatened, legal claims or litigation, in each case, relating to antitrust, anti-competition, or Anti-Corruption Law violations.

10.2.9 To uniQure's Knowledge, neither uniQure nor any of its Affiliates, or its or their directors, officers, employees, distributors, agents, representatives, sales intermediaries, or other Third Parties acting on behalf of uniQure or any of its Affiliates has violated any applicable Anti-Corruption Laws in any material respect in the past five years.

10.2.10 To uniQure's Knowledge, neither uniQure nor any of its Affiliates, or its or their directors, officers, employees, distributors, agents, representatives, sales intermediaries, or other Third Parties acting on behalf of uniQure or any of its Affiliates has made any untrue statement of a material fact or fraudulent statement to any Governmental Authority or Institutional Review Board, or failed to disclose any material fact required to be disclosed to any Governmental Authority or Institutional Review Board, or has committed any act, made any statement or failed to make any statement that would reasonably be expected to provide a basis for the FDA to invoke its policy, "Fraud, Untrue Statements of Material Facts, Bribery, and Illegal Gratuities" set forth in 56 Fed. Reg. 46191 (Sept. 10, 1991) or for any other Governmental Authority to invoke any similar policy.

10.2.11 Neither uniQure nor any of its Affiliates is subject to any obligation or requirement arising under any injunction, consent decree, inspection report, warning letter, untitled letter, notice of violation letter, FDA Form 483, notice of adverse findings or other regulatory or administrative action issued by or entered into with any Governmental Authority or Institutional Review Board. uniQure and its Affiliates are not the subject of any pending or threatened enforcement or other action that asserts a lack of compliance or failure to comply in any material respect with any Applicable Law. uniQure, its Affiliates, and its Subcontractors hold all consents, registrations, licenses, and permits required under Applicable Law for the Development and Manufacturing of the Licensed Product (as conducted as of the Execution Date).

10.2.12 Each Licensed Product has been Developed in material compliance with all Applicable Laws, including all GLP and GCP.

10.3 Representations and Warranties of Partner. Partner represents and warrants to uniQure as of the Execution Date as follows:

10.3.1 There are no legal claims, judgments, or settlements against or owed by Partner or any of its Affiliates, or pending or, to Partner's Knowledge, threatened, legal claims or litigation, in each case, relating to antitrust, anti-competition, or Anti-Corruption Law violations, except as would not, individually or in the aggregate, reasonably be expected to have a material adverse effect on Partner's ability to perform its obligations under this Agreement.

10.3.2 To Partner's Knowledge, neither Partner nor any of its Affiliates, or its or their directors, officers, employees, distributors, agents, representatives, sales intermediaries, or other Third Parties acting on behalf of Partner or any of its Affiliates has violated any applicable Anti-Corruption Laws in any material

respect, in the past five years, except as would not, individually or in the aggregate, reasonably be expected to have a material adverse effect on Partner's ability to perform its obligations under this Agreement.

10.4 Compliance Covenants. Each Party covenants to the other Party that, in the course of performing its obligations or exercising its rights under this Agreement, it will comply with all Applicable Laws and, without limiting the foregoing:

10.4.1 it will not employ or engage, and if so employed and engaged, it will thereafter cease to use any Person who has been Debarred/Excluded (including any Subcontractor) or is the subject of any proceedings that could result in such Person being Debarred/Excluded;

10.4.2 it will not perform any actions that are prohibited by any Anti-Corruption Laws that are applicable to it; and

10.4.3 it will not, directly or indirectly, make any payment, or offer or transfer anything of value, or agree or promise to make any payment or offer or transfer anything of value, to a government official or government employee, to any political party or any candidate for political office, or to any other Third Party with the purpose of influencing decisions related to either Party or its Affiliates or its or their respective businesses in a manner that would violate Anti-Corruption Laws.

10.5 NO OTHER WARRANTIES. EXCEPT AS EXPRESSLY STATED IN THIS ARTICLE 10 (REPRESENTATIONS, WARRANTIES, AND COVENANTS), (A) NO REPRESENTATION, OR WARRANTY IS MADE OR GIVEN BY OR ON BEHALF OF UNIQURE OR PARTNER; (B) ALL OTHER REPRESENTATIONS OR WARRANTIES WHETHER ARISING BY OPERATION OF LAW OR OTHERWISE ARE EXPRESSLY EXCLUDED, INCLUDING ANY REPRESENTATIONS OR WARRANTIES OF MERCHANTABILITY, FITNESS FOR A PARTICULAR PURPOSE, TITLE, OR NON-INFRINGEMENT; AND (C) ANY INFORMATION PROVIDED BY EITHER PARTY OR ITS AFFILIATES IS MADE AVAILABLE ON AN "AS IS" BASIS WITHOUT REPRESENTATION OR WARRANTY WITH RESPECT TO COMPLETENESS, COMPLIANCE WITH REGULATORY STANDARDS OR REGULATIONS, OR FITNESS FOR A PARTICULAR PURPOSE OR ANY OTHER KIND OF WARRANTY WHETHER EXPRESS OR IMPLIED.

10.6 Additional Covenants Prior to the Effective Date.

10.6.1 **Actions Prior to the Effective Date**. Each Party covenants to the other Party that, from the Execution Date through the Effective Date, such Party shall not knowingly take any action and shall not knowingly omit to take any action if, as a result of such action or inaction, any of the representations and warranties of such Party (a) in the case of uniQure, in Section 10.1 (Representations and Warranties of Each Party) and Section 10.2 (Representations and Warranties of uniQure) or (b) in the case of Partner, in Section 10.1 (Representations and Warranties of Each Party) and Section 10.3 (Representations and Warranties of Partner), would have been untrue

or inaccurate as of the Execution Date had such action or omission by such Party occurred prior to such date.

10.6.2 **uniQure Additional Covenants Prior to the Effective Date.** Prior to the Effective Date, except (a) as may be required by Applicable Law, or (b) as Partner may otherwise approve in writing (with such approval not to be unreasonably withheld, conditioned or delayed), uniQure will (and it will cause its controlled Affiliates to) use Commercially Reasonable Efforts to conduct those Development and Manufacturing development activities with respect to the Licensed Products and the AAV5 NAb Assay included under the interim plan set forth in Schedule 10.6.2 (uniQure Interim Development and Manufacturing Development Plan) (the "**uniQure Interim Plan**") by the applicable target dates set forth in the uniQure Interim Plan, it being understood that the mere fact that any activity under the uniQure Interim Plan the applicable target date for which is later than the Effective Date is commenced but not completed by uniQure on the Effective Date shall not be deemed to be a breach of uniQure's obligations under this Section 10.6.2 (uniQure Additional Covenants Prior to the Effective Date). During the period prior to the Effective Date, uniQure will provide (and will cause its controlled Affiliates to provide) to Partner (i) copies of each of the reports and manuscripts referred to in the uniQure Interim Plan, promptly after such report or manuscript has been prepared, and (ii) such other reasonable reports and information relating to the Development or Manufacturing development of the Licensed Products as Partner reasonably may request in writing, as soon as reasonably practicable after such request, in each case, subject to Applicable Laws and it being understood that such reports, manuscripts, and information shall be the Confidential Information of uniQure and subject to the provisions of Article 9 (Confidentiality; Publications).

10.6.3 [*]. Prior to the Effective Date, at Partner's request, uniQure will reasonably assist Partner's efforts to [*] in conjunction with the Licensed Products and the AAV5 NAb Assay, in those countries in the Territory in which Partner intends to Commercialize the Licensed Products (including Commercialization with the AAV5 NAb Assay), as identified in the Commercialization Plan. At any time after the Effective Date, at Partner's reasonable request, [*] and (ii) reasonable personnel assistance to understand and use the foregoing.

10.6.4 **Partner Activities Prior to the Effective Date**. Prior to the Effective Date, without limiting any other rights Partner may have under this Agreement or otherwise, subject to Applicable Laws, Partner will use Commercially Reasonable Efforts to conduct planning activities it reasonably determines are necessary to prepare for Commercialization of the Licensed Products following the Effective Date. Upon uniQure's reasonable request, Partner will provide uniQure with periodic updates regarding the progress of such activities to the extent permitted by Applicable Laws and it being understood that such information provided by Partner shall be the Confidential Information of Partner and subject to the provisions of Article 9 (Confidentiality; Publications).

10.7 **Compliance with Laws**. Partner understands and acknowledges that uniQure is subject to regulation by Governmental Authorities in the E.U., including the European Commission, and in the U.S., including the U.S. Department of Commerce and the U.S. Treasury Department's Office of Foreign Assets Control, which regulate the import, export, and diversion of certain products and technology from and to certain countries. Any and all obligations of uniQure to provide the Licensed Product, as well as any other technical information or assistance, and all rights on the part of Partner to perform its obligations hereunder, will be subject in all respects to such Applicable Law in the E.U. and the U.S. as will from time to time govern the license and delivery of technology and products abroad by Persons subject to the jurisdiction of the European Union or United States, as applicable, including, with respect to the E.U., Regulation (EC) No 428/2009 and, with respect to the U.S., regulations promulgated under Executive Order No. 12924 of August 19, 1994 issued pursuant to the President's authority under the International Emergency Economic Powers Act, Title 50 U.S. C., Chapter 35, Section 1701 et seq. and those contained in Title 31, Part 500 of the U.S. Code of Federal Regulations. Partner will cooperate with uniQure including providing required documentation, in order to comply with any and all Applicable Law in the E.U. and the U.S. Both Parties will cooperate with the other as reasonably required, including providing required documentation, in order to comply with all Applicable Law in the E.U. and the U.S. governing exports that are applicable to the Parties and products in question.

10.8 **Limitation on Claims**. Except in the case of any fraud or intentional misrepresentation by a Party, and without limiting a Party's rights under Article 11 (Indemnification): (a) the representations and warranties of each of the Parties contained in Section 10.1 (Representations and Warranties of Each Party) (other than Section 10.1.1, Section 10.1.2 and Section 10.1.4), Section 10.2 (Representations and Warranties of uniQure) (other than in Section 10.2.1 and Section 10.2.2), and Section 10.3 (Representations and Warranties of Partner), in each case, will survive until the date that is [*] after the Effective Date; (b) the representations and warranties of each of the Parties contained in Section 10.1.1, Section 10.1.2, and Section 10.1.4 and of uniQure in Section 10.2.1 and Section 10.2.2 will survive until the date that is [*] following the Execution Date; (c) no claim may be made or suit instituted alleging breach or seeking indemnification pursuant to Article 11 Indemnification) for any breach of, or inaccuracy in, any representation or warranty contained in Section 10.1 (Representations and Warranties of Each Party), Section 10.2 (Representations and Warranties of uniQure), or Section 10.3 (Representations and Warranties of Partner) unless a written notice is provided to the Indemnifying Party at any time prior to the date that is [*] after the expiration of the relevant survival period as set forth in clause (a) or (b) above; and (d) after the expiration of the time period for bringing claims for breach of representation or warranty as set forth in clause (c) above, a Party may not bring any claim against the other Party arising from or relating to such other Party's breach of such representation or warranty.

ARTICLE 11
INDEMNIFICATION

11.1 **By Partner**. Partner will indemnify and hold harmless uniQure and its Affiliates, and their respective directors, officers, employees, successors, heirs and assigns, and agents

(individually and collectively, the "**uniQure Indemnitees**") from and against all Losses incurred in connection with any Third Party Claims to the extent arising from or relating to (a) the Exploitation of any Licensed Product by or on behalf of Partner or any of its Affiliates, Sublicensees, or Subcontractors, including, after the Manufacturing Responsibility Cutover Date, product liability claims arising from Licensed Product Manufactured by or on behalf of Partner or any of its Affiliates, Sublicensees, or Subcontractors or from any other Manufacturing activities for which Partner or any of its Affiliates, Sublicensees, or Subcontractors has responsibility, (b) Partner's actions (or omissions) in the performance of its obligations with respect to Regulatory Submissions or interactions with Regulatory Authorities, in each case, as the Regulatory Responsible Party, (c) the gross negligence or willful misconduct of Partner or any of its Affiliates, Sublicensees, or Subcontractors, (d) the fraud of Partner or any of its controlled Affiliates, (e) Partner's breach of any of its representations, warranties, covenants, or obligations set forth in or entered into pursuant to this Agreement; *provided* that, solely with respect to indemnification by Partner under this Section 11.1 (By Partner) for Third Party Claims, for purposes of determining whether any breach of any representation or warranty made by Partner in Section 10.3 (Representations and Warranties of Partner) has occurred and the amount of Losses resulting therefrom, arising in connection therewith, or relating thereto, the terms "material adverse effect" and other similar qualifications based upon materiality will be disregarded and given no effect, (f) the failure of Partner or any of its Affiliates, Sublicensees, or Subcontractors to abide by any Applicable Law, or (g) any claim or demand from any employee or contractor of Partner or any of its Affiliates who is an inventor of any Joint Technology with respect to the ownership thereof, in each case of clauses (a) through (g) above, except to the extent such Third Party Claims arise out of a uniQure Indemnitee's gross negligence, willful misconduct, or fraud, breach of this Agreement, or failure to abide by any Applicable Law.

11.2 **By uniQure**. uniQure will indemnify and hold harmless Partner, its Affiliates, and their directors, officers, employees, successors, heirs and assigns, and agents (individually and collectively, the "**Partner Indemnitees**") from and against all Losses incurred in connection with any Third Party Claims to the extent arising from or relating to (a) the Development or Manufacture of any Licensed Product by or on behalf of uniQure or any of its Affiliates, licensees (not including Partner or its Affiliates, Sublicensees, or Subcontractors), Sublicensees, or Subcontractors, including product liability claims arising from such Development or Manufacture, (b) the gross negligence or willful misconduct of uniQure or any of its Affiliates, licensees (not including Partner or its Affiliates, Sublicensees, or Subcontractors), Sublicensees, or Subcontractors, (c) the fraud of uniQure or any of its controlled Affiliates, (d) uniQure's breach of any of its representations, warranties, covenants, or obligations set forth in or entered into pursuant to this Agreement, (e) the failure of uniQure or any of its Affiliates, licensees (not including Partner or its Affiliates, Sublicensees, or Subcontractors), or Subcontractors to abide by any Applicable Law, or (f) any claim or demand from any employee or contractor of uniQure or any of its Affiliates who is an inventor of any Joint Technology with respect to the ownership thereof, in each case of clauses (a) through (f) above, except to the extent such Third Party Claims arise out of any of a Partner Indemnitee's gross negligence, willful misconduct, or fraud, breach of this Agreement, or failure to abide by any Applicable Law.

11.3 **Indemnification Procedure**. If either Party is seeking indemnification under Section 11.1 (Indemnification; By Partner) or Section 11.2 (Indemnification; By uniQure) (the "**Indemnified Party**"), then it will inform the other Party (the "**Indemnifying Party**") of the Third Party Claim giving rise to such indemnification obligations within [*] after receiving written notice of the Third Party Claim (it being understood and agreed, however, that the failure or delay by an Indemnified Party to give such notice of a Third Party Claim will not affect the Indemnifying Party's indemnification obligations hereunder except to the extent the Indemnifying Party will have been actually and materially prejudiced as a result of such failure or delay to give notice). The Indemnifying Party will have the right to assume, with counsel of its choice, the defense of any such Third Party Claim for which it is obligated to indemnify the Indemnified Party. The Indemnified Party will cooperate with the Indemnifying Party and the Indemnifying Party's insurer as the Indemnifying Party may reasonably request, and at the Indemnifying Party's cost and expense. The Indemnified Party will have the right to participate, at its own expense and with counsel of its choice, in the defense of any Third Party Claim that has been assumed by the Indemnifying Party. Neither Party will have the obligation to indemnify the other Party in connection with any settlement made without the first Party's written consent, which consent will not be unreasonably withheld. The Indemnifying Party will not admit liability of the Indemnified Party without the Indemnified Party's prior written consent, which consent may be withheld in the Indemnified Party's reasonable discretion. If the Parties cannot agree as to the application of Section 11.1 (Indemnification; By Partner) or Section 11.2 (Indemnification; By uniQure) as to any Third Party Claim, pending resolution of the Dispute pursuant to Article 14 (Dispute Resolution), then the Parties may conduct separate defenses of such Third Party Claims, with each Party retaining the right to claim indemnification from the other Party in accordance with Section 11.1 (Indemnification; By Partner) or Section 11.2 (Indemnification; By uniQure), as applicable, upon resolution of the underlying Third Party Claim.

11.4 **Insurance**. Each Party will procure and maintain during the Term of this Agreement and until the later of: (a) [*] after termination or expiration of this Agreement, or (b) the date that all statutes of limitation covering claims or suits that may be instituted for personal injury based on the sale or use of a Licensed Product have expired, commercial general liability insurance from a minimum of "A-" AM Bests rated insurance company or insurer reasonably acceptable to the other Party, including product liability or clinical trials, if applicable, with coverage limits of not less than $[*] per occurrence and $[*] in the aggregate. Such policies will add the other Party and its Affiliates as additional insureds and provide a waiver of subrogation in favor of the other Party and its Affiliates. Such insurance policies will be primary and non-contributing with respect to any other similar insurance policies available to the other Party or its Affiliates. Any deductibles for such insurance will be assumed by the Party maintaining such insurance. Each Party will provide to the other Party with evidence of such insurance (i) promptly following the execution by both Parties of this Agreement, (ii) upon a Party's request to the other Party, and (iii) prior to expiration of any one coverage. Each Party will provide the other Party with written notice at least [*] prior to the cancellation or non-renewal of, or material changes in, such insurance. Such insurance will not be construed to create a limit on Partner's or uniQure's respective liability with respect to its indemnification obligations under this Article 11 (Indemnification).

ARTICLE 12
INTELLECTUAL PROPERTY

12.1 Inventions.

12.1.1 **Ownership**. As between the Parties, (a) uniQure will solely own all uniQure Technology and all Know-How developed or invented in the performance of activities under this Agreement solely by uniQure's or its Affiliates', licensees', or Subcontractors' employees, agents, or independent contractors, or any Persons contractually required to assign such Know-How to uniQure or any Affiliate of uniQure and all Patent Rights that Cover any Inventions in such Know-How, but excluding Joint Technology, (b) Partner will solely own all Partner Technology, including all Know-How developed or invented in the performance of activities under this Agreement solely by Partner or its Affiliates', licensees', Sublicensees', or Subcontractors' employees, agents, or independent contractors, or any Persons contractually required to assign such Know-How to Partner or any Affiliate of Partner and all Patent Rights that Cover any Inventions in such Know-How, but excluding Joint Technology, and (c) the Parties will jointly own all Joint Technology. Subject only to the rights expressly granted to the other Party under this Agreement, each Party, as between such Party and the other Party, will own all rights, title, and interests in and to any Know-How that is invented, conceived, discovered, created, or otherwise developed by or on behalf of such Party (or its Affiliates or its Sublicensees) under or in connection with this Agreement, whether or not patented or patentable, and any and all Patent Rights and other intellectual property rights with respect thereto. For purposes of determining ownership of Inventions under this Agreement, all determinations of inventorship under this Agreement will be made in accordance with U.S. patent law.

12.1.2 **Disclosure**. Each Party will disclose to the other Party, at least [*] in advance of each JSC meeting, all Inventions that it develops or invents in the performance of activities under this Agreement, whether solely or jointly with others (in any event, prior to the filing of any patent application with respect to such Inventions), including all invention disclosures or other similar documents submitted to a Party by its or its Affiliates' employees, agents, or independent contractors relating thereto that may reasonably be anticipated to be material to the other Party. Each Party will also promptly respond to reasonable requests from the other Party for additional information relating thereto.

12.2 Practice Under and Other Use of Joint Technology. Subject to the rights granted under and the restrictions set forth in this Agreement (including the licenses granted under Article 2 (Licenses) and the restrictions set forth in Section 2.6 (Exclusivity Covenant)), each Party will be entitled to the free use and enjoyment of all Joint Technology and neither Party will have any obligation to account to the other Party for profits, or to obtain any approval of the other Party to license, assign, or otherwise exploit its interest in any Joint Technology by reason of joint ownership thereof. Each Party hereby waives any right it may have under the Applicable Law of any jurisdiction to require any such approval or accounting. To the extent any further consent is required to enable a Party to so license or exploit its interest

in the Joint Technology, the other Party will grant consent promptly upon request. Without limitation, each Party will cooperate with the other Party if the Parties determine to apply for U.S. or foreign patent protection for any Joint Technology and will obtain the cooperation of the individual inventors of any such Joint Technology.

12.3 **CREATE Act**. Notwithstanding any provision to the contrary set forth in this Agreement, if a Party wishes to invoke the Cooperative Research and Technology Enhancement Act, 35 U.S.C. § 102(c) (the "**CREATE Act**") when exercising its rights under this Agreement, then it will notify the other Party and if agreed by the other Party, then the Parties will cooperate and coordinate their activities with respect to any filings or other activities in support thereof. The Parties acknowledge and agree that this Agreement is a "joint research agreement" as defined in the CREATE Act.

12.4 **Patent Prosecution**.

12.4.1 **uniQure Platform Patent Rights and uniQure Manufacturing Patent Rights**.

(a) **Right to Prosecute**. Except as set forth in Section 12.5.2(b) (Partner First Right), as between the Parties, uniQure will have the sole right (subject to Section 12.4.1(b) (Review and Consult)) to control the Patent Prosecution of all uniQure Platform Patent Rights and uniQure Manufacturing Patent Rights, in each case, throughout the Territory at its sole cost and expense.

(b) **Review and Consult**. uniQure will consult with Partner and keep Partner reasonably informed of the Patent Prosecution of the uniQure Platform Patent Rights and will provide Partner with all material correspondence received from any patent authority in the Territory in connection therewith. In addition, uniQure will provide Partner with drafts of all proposed material filings and correspondence to any patent authority in its respective territory in connection with the Patent Prosecution of the uniQure Platform Patent Rights and uniQure Manufacturing Specified Patent Rights for Partner's review and comment prior to the submission of such proposed filings and correspondence. uniQure will consider Partner's comments on Patent Prosecution of such Patent Rights in good faith and will incorporate such comments where appropriate, but will have final decision-making authority under this Section 12.4.1(b) (Review and Consult).

12.4.2 **Product Patent Rights**.

(a) **Right to Prosecute**. Except as set forth in Section 12.5.2(b) (Partner First Right), uniQure will have the first right to control the Patent Prosecution of any uniQure Product Patent Rights in the Territory, at uniQure's sole cost and expense. uniQure will use reasonable efforts in connection with such Patent Prosecution to obtain and maintain any granted claims.

(b) **Review and Consult**. uniQure will consult with Partner and keep Partner reasonably informed of the Patent Prosecution of the uniQure Product Patent Rights, including discussing further claims that may be filed in the

patent families thereof and will provide Partner with all material correspondence received from any patent authority in the Territory in connection therewith. In addition, uniQure will provide Partner with drafts of all proposed material filings and correspondence to any patent authority in its respective territory in connection with the Patent Prosecution of the uniQure Product Patent Rights for Partner's review and comment prior to the submission of such proposed filings and correspondence. Further, uniQure will notify Partner if it is contemplating ceasing Patent Prosecution of any of the uniQure Product Patent Rights. uniQure will consider Partner's comments on Patent Prosecution of such uniQure Product Patent Rights in good faith and will incorporate such comments where appropriate, but uniQure will have final decision-making authority under this Section 12.4.2(b) (Review and Consult).

(c) **Abandonment**. If uniQure decides that it is no longer interested in the Patent Prosecution of a particular uniQure Product Patent Right in the Territory, then it will promptly (and reasonably in advance of any applicable deadline) provide written notice to Partner of such decision. Partner may in its discretion, upon written notice to uniQure, assume the Patent Prosecution of such Patent Right. In such event, Partner will be responsible for 100% of the costs and expenses incurred with respect to the Patent Prosecution of such Patent Rights in the Territory, and such Patent Rights will no longer be considered uniQure Royalty Patent Rights for the purposes of this Agreement.

12.4.3 **Joint Patent Rights**.

(a) **Right to Prosecute**. Partner will have the first right to control the Patent Prosecution of any Joint Patent Rights, in each case, in the Territory, at Partner's sole cost and expense.

(b) **Review and Consult**. Partner will consult with uniQure and keep uniQure reasonably informed of the Patent Prosecution of the Joint Patent Rights and will provide uniQure with all material correspondence received from any patent authority in the Territory in connection therewith. In addition, Partner will provide uniQure with drafts of all proposed material filings and correspondence to any patent authority in its respective territory in connection with the Patent Prosecution of the Joint Patent Rights for uniQure's review and comment prior to the submission of such proposed filings and correspondence. Further, Partner will notify uniQure if it is contemplating ceasing Patent Prosecution of any of the Joint Patent Rights. Partner will consider uniQure's comments on Patent Prosecution of such Joint Patent Rights in good faith and will incorporate such comments where appropriate, but Partner will have final decision-making authority under this Section 12.4.2(b) (Review and Consult).

(c) **Abandonment**. If Partner decides that it is no longer interested in the

Patent Prosecution of a particular Joint Patent Right in the Territory, then it will promptly (and reasonably in advance of any applicable deadline) provide written notice to uniQure of such decision. uniQure may, upon written notice to Partner, assume the Patent Prosecution of such Joint Patent Right. In such event, uniQure will be responsible for 100% of the costs and expenses incurred with respect to the Patent Prosecution of such Joint Patent Rights in the Territory.

12.5 Patent Enforcement.

12.5.1 **Notice**. Each Party will notify the other Party within [*] after becoming aware of any alleged or threatened infringement by a Third Party product that is competitive with any Licensed Product of any of the (a) uniQure Patent Rights Covering a Licensed Product or (b) Joint Patent Rights, ("**Product Infringement**"). The Party with the first right to bring and control any legal action to enforce the uniQure Patent Rights or other Joint Patent Rights, as applicable, under this Section 12.5 (Patent Enforcement) will be referred to herein as the "**Controlling Party**."

12.5.2 **First Right and Step-In for Product Infringement**.

(a) **Partner First Right – Enforcement**. Subject to Section 12.5.2(c) (uniQure Blocking Right) Partner will have the first right to bring and control, at its sole cost and expense, any legal action or proceeding to enforce the uniQure Product Patent Rights, uniQure Manufacturing Specified Patent Rights, uniQure Platform Patent Rights, (in each case, other than as set forth in Section 12.5.3 (uniQure Sole Right)), and Joint Patent Rights against any Product Infringement in the Field in the Territory as it reasonably determines appropriate, and Partner will consider in good faith the interests of uniQure in such enforcement of such Patent Rights.

(b) **Partner First Right – Invalidity**. Partner will have the first right to control, at its sole cost and expense, any legal action or proceeding to defend any uniQure Product Patent Rights, uniQure Manufacturing Specified Patent Rights, uniQure Platform Patent Rights, and Joint Patent Rights against any declaratory judgments or adversarial proceedings, including, and notwithstanding anything to the contrary in this Agreement, IPRs, PGRs, re-examinations, oppositions or equivalent actions, challenging the validity, scope or enforceability or alleging the non-infringement of such Patent Rights as Partner reasonably determines appropriate, solely to the extent the foregoing arise as a result of any legal action or proceeding by Partner pursuant to Section 12.5.2(a) (Partner First Right – Enforcement) and are not part of Patent Prosecution activities.

(c) **uniQure Blocking Right.** Partner's right under Section 12.5.2(a) (Partner First Right – Enforcement) to enforce the uniQure Platform Patent Rights or uniQure Manufacturing Specified Patent Rights requires the prior written consent of uniQure, which may not be unreasonably withheld. If uniQure

does not consent, then such uniQure Platform Patent Right or uniQure Manufacturing Specified Patent Right will cease to be a taken into account for the purposes of the calculation of the Royalty Term in Section 8.3.1 (Royalty Payments) and will be deemed to be expired for the purposes of the royalty step-down in Section 8.3.2(a) (Patent Expiration Step-Down).

(d) **Step-In Rights**. If the Controlling Party or its designee fails to abate the applicable Product Infringement in the Territory; or file an action to abate such Product Infringement in the Territory within [*] after a written request from the other Party to do so (or sooner, if reasonably necessary under the circumstances) or defend any action or proceeding in Section 12.5.2(b) (Partner First Right – Invalidity), or if the Controlling Party discontinues the prosecution of any such action or proceeding after filing without abating such infringement (or discontinues any such defense), then, in either case, the other Party will have the right to enforce the applicable Patent Rights against such Product Infringement in the Territory or defend such action or proceeding as it reasonably determines appropriate, which right for Product Infringement will be limited to Product Infringements in the Field if Partner is the non-Controlling Party; *provided* that (i) the Controlling Party does not provide reasonable rationale for not doing so or continuing to do so (including, for Product Infringement, a substantive concern regarding counter-claims by the infringing Third Party); (ii) the other Party will not enter into any settlement admitting the invalidity of, or otherwise impairing, any such Patent Rights without the prior written consent of the Controlling Party; and (iii) for a Product Infringement action, Partner will not have any right to enforce any uniQure Platform Patent Right if there are any uniQure Product Patent Rights, uniQure Manufacturing Specified Patent Rights or Joint Patent Rights that can be enforced against the applicable Product Infringement (regardless of whether or not uniQure seeks to take action against the applicable Product Infringement).

12.5.3 **uniQure Sole Right**. uniQure will have the sole right to bring and control, at its sole cost and expense, any legal action or proceeding to enforce (a) the uniQure Manufacturing Patent Rights and (b) any uniQure Patent Rights not included in the uniQure Royalty Patent Rights, in each case in the Territory.

12.5.4 **Recoveries**. Any recoveries resulting from an enforcement action relating to a claim of Product Infringement in the Territory will be first applied against payment of each Party's costs and documented out-of-pocket Third Party expenses in connection therewith. Any such recoveries after such application will be shared between the Parties as determined by the Parties at the applicable time based on their respective economic interests in such recovery, *provided* that (i) uniQure will retain [*]% of any remaining recoveries (after the above application) from the enforcement of any uniQure Patent Rights not included in the uniQure Royalty Patent Rights pursuant to clause (b) of Section 12.5.3 (uniQure Sole Right), and (ii) Partner will retain [*]% of any remaining recoveries (after the above application) from the enforcement of any uniQure Royalty Patent Rights that lose such status

due to their abandonment by uniQure pursuant to Section 12.4.3(c) (Abandonment).

12.6 Infringement of Third Party Rights.

12.6.1 **Notice**. If any Licensed Product used or sold by Partner or its Affiliates or Sublicensees becomes the subject of a Third Party's claim or assertion of infringement of a Patent Right or other rights in the Territory that are owned or controlled by such Third Party, then Partner will promptly notify uniQure within [*] after receipt of such claim or assertion and will include in such notice a copy of any summons or complaint (or the equivalent thereof) received regarding the foregoing. Thereafter, the Parties will promptly meet to consider the claim or assertion and the appropriate course of action.

12.6.2 **Defense**. Partner will be solely responsible for the defense of any infringement claims referenced in Section 12.6.1 (Notice) brought against Partner, at Partner's cost and expense; *provided* that Partner will not agree to any settlement, consent to judgment, or other voluntary final disposition in connection with such defense action without uniQure's prior written consent if such settlement, consent to judgment, or other voluntary final disposition would (a) result in the admission of any liability or fault on behalf of uniQure, (b) result in or impose any payment obligations upon uniQure, or (c) subject uniQure to an injunction or otherwise limit uniQure's ability to take any actions or refrain from taking any actions under this Agreement or with respect to any Licensed Product. Partner will keep uniQure informed on the status of such defense action, and uniQure will have the right, but not the obligation, to participate and be separately represented in such defense action at its sole option and at its own expense. Partner may credit [*]% of any payments required to license any Patent Rights from any Third Party to resolve an infringement claim related to the Licensed Product against any Royalties due to uniQure under this Agreement, subject to Section 8.3.2(d) (Royalty Reductions Floor). Notwithstanding the foregoing or anything to the contrary set forth in this Agreement, uniQure will be solely responsible for the defense of any infringement claim, at uniQure's cost and expense, brought with respect to Partner's or uniQure's (or their Affiliates' and its and their respective Subcontractors' and Sublicensees') use of any step or method of Manufacturing provided by uniQure to Manufacture any Licensed Product, *provided* that uniQure will not agree to any settlement, consent to judgment, or other voluntary final disposition in connection with such defense action without Partner's prior written consent if such settlement, consent to judgment, or other voluntary final disposition would (i) result in the admission of any liability or fault on behalf of Partner, its Affiliates or its or their respective Subcontractors or Sublicensees, (ii) result in or impose any payment obligations upon Partner or such Persons, or (iii) subject Partner or such Persons to an injunction or otherwise limit Partner's or such Persons' ability to take any actions or refrain from taking any actions under this Agreement or with respect to any Licensed Product. uniQure will keep Partner informed on the status of such defense action, and Partner will have the right, but not the obligation, to participate and be separately represented in such defense action at its sole option and at its own

expense. uniQure will bear 100% of any payments required to license any Patent Rights from any Third Party to resolve any such infringement claim.

12.7 **Patent Listings**. With respect to the FDA's Purple Book and patent listings in any patent listing system established by any applicable Regulatory Authority in the Territory during the Term that is similar to the FDA Purple Book, for issued patents for any Licensed Product in the Field, the Parties will agree which patents, if any, to list in the FDA's Purple Book and such other patent listing (a) prior to the submission to such applicable Regulatory Authority of the first and any subsequent MAA for such Licensed Product in the Field in the applicable country in the Territory, and (b) within [*] the receipt from such Regulatory Authority of the first and any subsequent Regulatory Approval in the Field in the Territory for such Licensed Product in such country.

12.8 **Patent Term Extensions**. With respect to any system for extending the term of Patent Rights in the Territory established by any applicable Regulatory Authority during the Term that is similar to the patent term extension system in the U.S. (and in the U.S.), Partner will be solely responsible for making all decisions regarding patent term extensions in the Territory, including supplementary protection certificates and any other extensions that are now available or become available in the future, that are applicable to uniQure Product Patent Rights or Joint Patent Rights licensed hereunder and that become available directly as a result of the Regulatory Approval of a Licensed Product in the Territory; *provided* that Partner will consult with uniQure with respect to such decisions and will consider in good faith the comments of uniQure thereon.

12.9 **Cooperation**. Each Party will provide the other Party all reasonable assistance and cooperation in connection with all activities under Sections 12.4 (Patent Prosecution), 12.5 (Patent Enforcement) and 12.6 (Infringement of Third Party Rights), including, as applicable, providing any necessary powers of attorney, executing other required documents, cooperating in discovery, keeping the other party reasonably informed of the status of the action or proceeding, taking good faith consideration of comments from the other party, and joining as a party to the action or proceeding if required by Applicable Law to pursue or maintain same. Each Party, at its sole expense and discretion, may select and involve external counsel of its choice in connection with performing the foregoing and exercising its rights under the above sections.

12.10 **Privilege**. The Parties intend and agree that any disclosures in connection with the activities under Section 12.4 (Patent Prosecution), Section 12.5 (Patent Enforcement) and Section 12.6 (Infringement of Third Party Rights), (a) must be treated by the receiving Party as confidential and in a manner that preserves all applicable privileges to the maximum extent possible; (b) are not deemed to waive any applicable privilege (including attorney-client privilege, attorney work product privilege and joint defense privilege) available to either or both Parties; and (c) are made in connection with the joint prosecution or joint defense (as applicable) and common legal interest of the Parties. If requested by either Party, the Parties will execute a "common interest agreement", "joint defense agreement" or similar agreement as appropriate to attempt to preserve all applicable privileges with respect to the above disclosures.

12.11 **Patent Marking**. Partner will mark all Licensed Product in accordance with the applicable patent marking laws and will require all of its Affiliates and Sublicensees to do the same, and any of them may use virtual marking to do so. To the extent permitted by Applicable Law, Partner will use reasonable efforts to indicate on the product packaging, advertisement and promotional materials that such Licensed Product is in-licensed from uniQure.

12.12 **Termination of Rights.** Partner's rights under Section 12.4 (Patent Prosecution), Section 12.5 (Patent Enforcement), Section 12.7 (Patent Listings), and Section 12.8 (Patent Term Extension) will terminate upon the earlier of (a) Partner's delivery to uniQure of notice of termination pursuant to Section 13.2.1 (Termination Without Cause), or (b) the effective date of the termination of this Agreement.

ARTICLE 13
TERM AND TERMINATION

13.1 **Term**. Unless earlier terminated in accordance with Section 13.2 (Termination), and without limiting or modifying Section 8.3.1 (Royalty Payments) with respect to expiration of the Royalty Term, this Agreement will be effective as of the Effective Date, and will continue, on a country-by-country basis, in full force and effect until the expiration of the Royalty Term in such country and will expire in its entirety upon the expiration of the final Royalty Term (the "**Term**"). Following expiration of the Royalty Term in a country, the license grants in Section 2.1 (License Grants to Partner, Licensed Products and AAV5 NAb Assay) will remain in place for a period of [*], but will substitute the phrase "uniQure Technology other than the uniQure Royalty Patent Rights" in place of the term "uniQure Technology." Following the expiration of such [*] period, the license grants in Section 2.1 (License Grants to Partner, Licensed Products and AAV5 NAb Assay) will become non-exclusive, fully-paid, royalty-free, perpetual, and irrevocable for such country.

13.2 **Termination**.

13.2.1 **Termination Without Cause**. Prior to receipt of the first Regulatory Approval of any Licensed Product in the first Major Country, Partner may terminate this Agreement in its entirety for convenience and without cause upon [*] prior written notice to uniQure. After receipt of the first Regulatory Approval of any Licensed Product in the first Major Country, Partner may terminate this Agreement in its entirety for convenience and without cause upon [*] prior written notice to uniQure.

13.2.2 **Termination For Cause**.

(a) **Defaults**. If a Party materially breaches any of its material obligations (including, for the avoidance of doubt, any of its obligations that are effective prior to the Effective Date as set forth in Section 15.1 (Effective Date)), excluding any breach or alleged breach by a Party to use Commercially Reasonable Efforts as required under this Agreement (as such breach of alleged breach will be governed by the provisions of Section 13.2.2(b) below) (a "**Default**"), then the non-defaulting Party may deliver

notice to the defaulting Party describing in reasonable detail the alleged Default in question, including the specific alleged material breach(es) of such material obligations by the defaulting Party (a "**Default Notification**"). For any Default arising from a failure to make an undisputed payment set forth in this Agreement, the allegedly defaulting Party will have [*] from the receipt of the applicable Default Notification to cure such payment Default. For all other Defaults, the allegedly defaulting Party will have [*] from the date of the Default Notification to cure such Default, *provided* that if such Default is not reasonably capable of cure within such [*] period, but is capable of cure within [*] from the date of such Default Notification (or such other period as may be agreed by the non-defaulting Party), then the defaulting Party may submit in writing to the non-defaulting Party, within [*] of such Default Notification, a reasonable cure plan that is reasonably acceptable to the non-defaulting Party, to remedy such Default as soon as reasonably possible and in any event prior to the end of such [*] period (or such other period as may be agreed by the non-defaulting Party), and, upon such submission, the [*] cure period will be automatically extended for so long as the defaulting Party continues to use reasonable efforts to cure such Default in accordance with such cure plan, but for no more than [*] (or such other period as may be agreed by the non-defaulting Party). If the defaulting Party fails to cure a Default within the applicable cure period or in accordance with the applicable cure plan as set forth above, then the non-defaulting Party may terminate this Agreement effective upon written notice of termination to the other Party.

(b) **CRE Defaults**. If a Party (the "**Alleging Party**") believes in good faith that the other Party (the "**Alleged Party**") has not used Commercially Reasonable Efforts as required under this Agreement (a "**CRE Default**"), then the Alleging Party may deliver notice to the Alleged Party describing in reasonable detail the alleged CRE Default in question, including the specific alleged failure(s) of the Alleged Party to use such Commercially Reasonable Efforts (a "**CRE Default Notification**"). Within [*] after receipt of a CRE Default Notification, the Alleged Party will either (x) submit in writing to the Alleging Party a good faith cure plan setting out the rectifying steps proposed to be taken by the Alleged Party to cure its alleged failure(s) to use Commercially Reasonable Efforts, including a timeline for doing so (a "**CRE Cure Plan**"), or (y) submit in writing to the Alleging Party a good faith explanation in reasonable detail as to why the Alleged Party disagrees with the alleged CRE Default (a "**CRE Explanation**").

(i) **CRE Cure Plans**. If the Alleged Party timely submits a CRE Cure Plan in accordance with the foregoing clause (b), then within [*] thereafter the Alleging Party, having considered the CRE Cure Plan in good faith, will respond in writing to the Alleged Party either accepting such CRE Cure Plan, proposing to modify such CRE Cure Plan (in which case the proposed modifications will be specified in

reasonable detail), or rejecting such CRE Cure Plan. If the Alleging Party accepts the CRE Cure Plan (or does not timely respond), then the Alleged Party will implement the CRE Cure Plan in accordance with its terms; *provided* that, if the Alleged Party fails to implement the CRE Cure Plan in any material respect, then the Alleging Party may terminate this Agreement upon [*] written notice to the Alleged Party. If the Alleging Party timely responds and proposes to modify the CRE Cure Plan, then the Parties will discuss in good faith the proposed modification for a period of [*], and if the Parties agree upon a modified CRE Cure Plan during such [*] period, then such modified CRE Cure Plan will constitute an "accepted" CRE Cure Plan hereunder and the provisions in the immediately preceding sentence with respect to an accepted CRE Cure Plan will apply. If the Parties do not agree on a modified CRE Cure Plan during such [*] period, then the CRE Cure Plan will constitute a "rejected" CRE Cure Plan hereunder and the provisions in the immediately following sentence with respect to a rejected CRE Cure Plan will apply. If the Alleging Party timely rejects the CRE Cure Plan, then the provisions of sub-clause (ii) below will apply.

(ii) **CRE Explanation**. If the Alleged Party timely submits a CRE Explanation in accordance with the foregoing clause (b), then within [*] thereafter the Alleging Party, having considered the CRE Explanation in good faith, will respond in writing to the Alleged Party either accepting or rejecting the CRE Explanation. If the Alleging Party accepts the CRE Explanation (or fails to timely respond), then the related CRE Default Notification will be deemed revoked and the Alleged Party will have no further obligation in respect thereof. If either (A) the Alleging Party timely rejects the CRE Explanation or (C) the Alleging Party timely rejects the CRE Cure Plan in accordance with the foregoing sub-clause (i), then the alleged CRE Default (including the matters that have transpired between the Parties pursuant to this Section 13.2.2(b) in respect thereof) will be referred to the Executive Officers for attempted resolution. If the Executive Officers are unable to resolve the alleged CRE Default within [*] after such matter is referred to them, then, upon the written request of either Party to the other Party, the alleged CRE Default will be subject to arbitration in accordance with Section 14.2 (Arbitration) (and, for the avoidance of doubt, the pre-arbitration negotiation procedure set forth in Section 14.1 (Pre-Arbitration Negotiation) will not apply) to determine whether or not such CRE Default occurred.

(iii) **Resolution by Arbitration**. If the alleged CRE Default is referred to arbitration in accordance with the foregoing sub-clause (ii), then:

(1) if the arbitrators determine that there was not any CRE Default by the Alleged Party, then the related CRE Default Notification will be deemed revoked and the Alleged Party will have no further obligation in respect thereof;

(2) if the arbitrators determinate that there was a CRE Default by the Alleged Party but a CRE Cure Plan was timely submitted by the Alleged Party and such CRE Cure Plan was a reasonable cure plan, then such CRE Cure Plan will constitute an "accepted" CRE Cure Plan and the provisions in the foregoing sub-clause (i) with respect to an accepted CRE Cure Plan will apply; or

(3) if the arbitrators determine that there was a CRE Default by the Alleged Party and either a CRE Cure Plan was not timely submitted by the Alleged Party or a CRE Cure Plan was timely submitted by the Alleged Party but such CRE Cure Plan was not a reasonable cure plan, then the Alleging Party may terminate this Agreement by [*] written notice to the Alleged Party.

(c) **Payment Adjustment in Lieu of Termination for Third Party Competitive Product**.

(i) If (i) Partner has the right to terminate this Agreement pursuant to, and in accordance with, clause (a) or clause (b) of Section 13.2.2 (Termination for Cause) as a result of a Default or CRE Default, respectively, of uniQure of any of its obligations under this Agreement and (ii) as a proximate cause of such Default (in the case of a termination right under Section 13.2.2(a)) or such CRE Default (in the case of a termination right under Section 13.2.2(b)), as the case may be, a Competitive Product of any Third Party achieves (x) [*] or (y) [*], it being understood that, unless uniQure expressly agrees or stipulates that such Default or CRE Default was the proximate cause of such eventuality, such question of proximate cause must be raised and determined in the same arbitration that determines such Default or CRE Default (except in the case of a Default of uniQure of its obligations under Section 2.6 (Exclusivity Covenant) where such proximate causation shall be presumed absent a specific determination to the contrary in such arbitration), then, without limiting its right to terminate this Agreement or to seek any other available remedies in connection with such Default or CRE Default, Partner may provide written notice to uniQure requiring the Parties to, in good faith, renegotiate any remaining Milestone Payments and the Royalty Rates as promptly as reasonably practicable after the provision of such notice, *provided* that such reduction [*].

(ii) If uniQure does not dispute the applicability of this Section 13.2.2(c) (Payment Adjustment in Lieu of Termination for Third Party Competitive Product) or if it is otherwise agreed or determined to be applicable pursuant to, and in accordance with, clause (a) or clause (b) of Section 13.2.2 (Termination for Cause) or Article 14 (Dispute Resolution), but the Parties fail to agree upon the renegotiated Milestone Payments and Royalty Rates within [*] after delivery of such notice, then such dispute regarding the renegotiated Milestone Payments and Royalty Rates will be resolved in accordance with the specified expedited arbitration procedures set forth in Section 14.7 (Specified Expedited Arbitrations) (and, for the avoidance of doubt, the pre-arbitration negotiation procedure set forth in Section 14.1 (Pre-Arbitration Negotiation) will not apply). After the resolution of the renegotiated Milestone Payments and Royalty Rates by the Parties' agreement under this clause (c) or (where a dispute has been referred to specified expedited arbitration) by the arbitrator's award in accordance with Section 14.7 (Specified Expedited Arbitrations), Partner may either: (A) accept such resolution (in which case such will be Partner's sole and exclusive remedy and Partner will be deemed to have waived its rights to terminate this Agreement pursuant to clause (a) or clause (b) of Section 13.2.2 (Termination for Cause), as applicable, and to seek any other available remedies in connection with such Default or CRE Default); or (B) terminate this Agreement pursuant to, and in accordance with, clause (a) or clause (b) of Section 13.2.2 (Termination for Cause), as applicable, and seek any other available remedies in connection with such Default or CRE Default, subject to the terms of this Agreement.

(d) Notwithstanding anything in this Agreement to the contrary and without limiting uniQure's other rights and remedies under the circumstances, uniQure does not have the right to terminate this Agreement under this Section 13.2.2 (Termination For Cause) due to (i) a breach by Partner of Section 12.1.2 (Disclosure); or (ii) any act or omission of a Subcontractor or Sublicensee, *provided* that Partner complies with its own obligations in Section 2.2.4 (Terms of Sublicensing and Subcontractor Agreements) or its termination obligation in the last sentence of Section 2.2.6 (Responsibility for Sublicensees and Subcontractors), unless and until the arbitration panel in an arbitration conducted in accordance with Article 14 (Dispute Resolution) rules that Partner has intentionally and materially breached such covenant by practicing an invention that infringes a claim in clause (i) of the definition of "Valid Claim" with respect to a uniQure Royalty Patent Right.

13.2.3 **Termination for Patent Challenge**. uniQure may terminate this Agreement in its entirety by providing written notice of termination to Partner if Partner or its Affiliates or Sublicensees (individually or in association with any Person) contests or assists a Third Party in contesting the scope, validity, or enforceability of any

uniQure Royalty Patent Right, in each case, anywhere in the world in any court, tribunal, patent office, arbitration proceeding, or other proceeding, including the U.S. Patent and Trademark Office and the U.S. International Trade Commission (a "**Patent Challenge**"), subject to the cure provision below and except if such activities are [*]. In the event of such a Patent Challenge, uniQure will provide prompt written notice of such Patent Challenge to Partner, and uniQure may terminate this Agreement by providing written notice of such termination to Partner, solely if Partner or its applicable Affiliates or Sublicensees do not, within [*] of receipt of such notice, (i) withdraw such Patent Challenge, or (ii) with respect to any Patent Challenge by any Sublicensee of Partner, if Partner does not provide such Sublicensee notice of termination of the sublicense agreement with such Sublicensee before the end of such notice period. If uniQure believes based on the advice of counsel that termination of this Agreement pursuant to this Section 13.2.3 (Termination for Patent Challenge) is not an available remedy under Applicable Law, it shall notify Partner in writing. If Partner responds within [*] that it disagrees with such belief, then uniQure may submit the issue to expedited arbitration in accordance with Section 14.6 (Expedited Arbitration). If uniQure initiates such arbitration and prevails in such arbitration on the foregoing issue, then in lieu of such termination right, if one accrues under this Section 13.2.3 (Termination for Patent Challenge), uniQure may instead [*] by providing written notice of such election to Partner. As used herein, a Patent Challenge includes: (a) filing an action under 28 U.S.C. §§ 2201-2202 seeking a declaration of invalidity or unenforceability of any such Patent Right; (b) filing, or joining in, a petition under 35 U.S.C. § 311 to institute *inter partes* review of any such Patent Right; (c) filing, or joining in, a petition under 35 U.S.C. § 321 to institute post-grant review of any such Patent Right or any portion thereof; (d) filing or commencing any opposition, nullity, or similar proceedings challenging the validity of any such Patent Right in any country or region; or (e) any foreign equivalent of clauses (a), (b), (c), or (d).

13.2.4 **Failure to Commercialize**. If Partner and its Affiliates and Sublicensees do not conduct any material Commercialization activities with respect to any Licensed Products in any of the Major Countries for a continuous period of longer than [*] at any time following the First Major Regulatory Approval and such failure to conduct any material Commercialization activities during such period is not: [*], then uniQure may deliver written notice to Partner of its intent to terminate this Agreement in its entirety, effective [*] after Partner's receipt of such notice, *provided* that, if Partner or any of its Affiliates or Sublicensees commences any material Commercialization activity with respect to any Licensed Product in any single Major Country during such [*] period, then this Agreement will not terminate upon the expiration of such [*] period. By way of illustration only, if, following the First Major Regulatory Approval, Partner or any of its Affiliates or Sublicensees conducted material Commercialization activities in a Major Country (for example, [*]) in a [*] period, then this Section 13.2.4 (Failure to Commercialize) will not apply with respect to such [*] period even if Partner or any of its Affiliates or Sublicensees had not conducted material Commercialization activities in any other Major Country in the same [*] period. For the avoidance of doubt, the existence of

this Section 13.2.4 (Failure to Commercialize) shall not be construed to limit Partner's obligations under Section 6.3 (Commercialization Diligence) prior to termination of this Agreement pursuant to this Section 13.2.4 (Failure to Commercialize) or any other termination provision in this Agreement.

13.2.5 **Full Force and Effect During Notice Period**. This Agreement will remain in full force and effect until the expiration of the applicable termination notice period. For clarity, if Partner or any of its Affiliates or Sublicensees achieve any Milestone Events during the termination notice period, then the corresponding Milestone Payment is accrued and Partner will remain responsible for the payment of such Milestone Payment even if the due date of such Milestone Payment occur, after the effective date of the termination.

13.3 Effect of Termination. In the event of any termination (but not expiration) of this Agreement, the following will apply:

13.3.1 **Licenses**. As of the effective date of termination of this Agreement, except as expressly set forth in this Agreement, all licenses and all other rights granted by uniQure to Partner hereunder under the uniQure Technology (other than as set forth below) will terminate and all sublicenses granted by Partner pursuant to Section 2.2 (Sublicensing and Subcontractors) with respect to the Licensed Products will also terminate. In addition, at uniQure's election upon such termination, uniQure will have, and Partner hereby grants and agrees to grant to uniQure, effective upon such termination, a worldwide, non-exclusive, perpetual, irrevocable, and sublicensable (through multiple tiers) license to uniQure under all Product Marks and all Patent Rights and Know-How Controlled by Partner or any of its Affiliates as of the effective date of termination that (a) Cover (with respect to Patent Rights) and (b) are necessary to Exploit (with respect to Product Marks and Know-How) the Licensed Product in the form that it exists of such effective date of termination (as such product may be modified by uniQure after the date of such termination, the "**Existing Product**"), solely to the extent necessary for uniQure to Exploit such Licensed Product. The foregoing license grant will be (i) [*] if this Agreement is terminated by uniQure for cause pursuant to Section 13.2.2 (Termination for Cause) or, if terminated for any other reason, [*] (A) [*] or (B) [*] and (ii) subject to the same conditions on sublicensing set forth in Section 2.2 (Sublicensing and Subcontractors) and the same royalty provisions in Sections 8.3.3 to 8.13 (inclusive), to be fully memorialized by the Parties within a reasonable time after the above termination date.

13.3.2 **Assignment of Agreements**. Partner will assign to uniQure in whole or in part as applicable any agreement with any Third Party to the extent such agreement is necessary for the Exploitation of the Existing Product, if permitted under such agreement (and will use reasonable efforts to seek any consent required from the applicable Third Party in connection with such an assignment, *provided* that Partner is not required to make any payments to such Third Party to procure such consent). If any such agreement (or portion thereof) cannot be assigned to uniQure, then upon uniQure's reasonable request, either (i) to the extent permitted under such

agreement, Partner will maintain (but is not obligated to renew or extend) such agreement (or portion thereof) and uniQure will pay to Partner 100% of all payments due to the applicable Third Party under any such agreement or (ii) Partner will cooperate reasonably with uniQure to procure an agreement directly with Third Party with respect to the Existing Product.

13.3.3 **Regulatory Submissions and Regulatory Approvals**. Partner will, and will cause its Affiliates and Sublicensees to, (a) no later than [*] after the effective date of termination of this Agreement, develop a plan for the assignment and transfer to uniQure or its designee all of Partner's rights, title, and interests in and to all Regulatory Submissions, Regulatory Approvals and Reimbursement Approvals for the Existing Product then owned or Controlled by Partner or any of its Affiliates or Sublicensees, (b) complete assignment and transfer promptly in accordance with such plan, and (c) to the extent assignment pursuant to clause (a) is delayed or is not permitted by the applicable Regulatory Authority, permit uniQure to cross-reference and rely upon any Regulatory Submissions, Regulatory Approvals, and Reimbursement Approvals filed by Partner with respect to such Existing Product. Partner will execute and deliver, or to cause to be executed and delivered, to uniQure or its designee such endorsements, assignments, commitments, acknowledgements, and other documents as may be necessary to assign, convey, transfer, and deliver to uniQure or its designee all of Partner's or its applicable Affiliate's or designee's rights, title, and interests in and to all such assigned Regulatory Submissions, Regulatory Approvals, and Reimbursement Approvals to uniQure, including submitting to each applicable Regulatory Authority or other Governmental Authority a letter or other necessary documentation (with copy to uniQure) notifying such Regulatory Authority or other Governmental Authority of, or otherwise giving effect to, the transfer of ownership to uniQure of all such assigned Regulatory Submissions, Regulatory Approvals, and Reimbursement Approvals. In addition, upon uniQure's written request, Partner will provide to uniQure copies of all material related documentation related to the Existing Product, including material non-clinical, preclinical, and clinical data related to the Existing Product that are held by or reasonably available to Partner or its Affiliates or Sublicensees. The Parties will discuss and establish appropriate arrangements with respect to safety data exchange, including with respect to any amendments to the SDE Agreement.

13.3.4 **Appointment as Exclusive Distributor**. If Partner is Commercializing the Existing Product in any country as of the effective date of termination, then, at uniQure's election (in its sole discretion) on a country-by-country basis, until such time as all Regulatory Approvals and Reimbursement Approvals with respect to such Existing Product in such country have been assigned and transferred to uniQure or its designee, Partner will appoint uniQure or its designee as its exclusive distributor of the Existing Products in such country and grant uniQure or its designee the right to appoint sub-distributors, to the extent not prohibited by any written agreement between Partner or any of its Affiliates and a Third Party.

13.3.5 **Assignment and Disclosure**. To the extent requested by uniQure, Partner will promptly upon such request:

(a) assign and transfer to uniQure or its designee all of Partner's rights, title, and interests in and to all clinical trial agreements (if any) and distribution agreements (to the extent assignable and not cancelled), confidentiality and other agreements; and provide copies of data and other Know-How (including commercial information) in Partner's Control, in each case, to the extent related to the Existing Products and that are necessary for the Exploitation of the Existing Product;

(b) assign to uniQure or its designee or amend to have uniQure assume Partner's rights and obligations therein, as either option is deemed appropriate in Partner's discretion, any agreements or arrangements with Third Party vendors (including distributors) with respect to the Existing Products or, to the extent any such Third Party agreement or arrangement is not assignable to uniQure for such scope, reasonably cooperate with uniQure to arrange for such distributor to continue to provide such services to Partner on uniQure's behalf for a reasonable time after termination of this Agreement with respect to such Existing Products to facilitate the orderly transition of all Commercialization and other activities then being performed by or on behalf of Partner or its Affiliates or Sublicensees for the Existing Products to uniQure or its designee, *provided* that, if Partner is required to make any payments to any Third Party to procure any consents, Partner's obligation to procure such consent is contingent on uniQure agreeing to bear 100% of any such payment;

(c) disclose to uniQure or its designee all documents, records, and materials to the extent related to the Existing Products that are Controlled by Partner or its Affiliates or Sublicensees and are necessary for uniQure to exercise its above rights relating to such Existing Products, which documents, records, and materials, in each case, will be deemed Partner's "Confidential Information" to the extent they also relate to Partner's or its Affiliates' other businesses or products and uniQure or its Affiliates and (sub)licensees may use the same to Exploit such Existing Products; and

(d) assign and transfer to uniQure or its designee all of Partner's rights, title, and interests in and to the copyright in any Promotional Materials that are solely related to the Existing Product (and provide a non-exclusive license to uniQure and its Affiliates and (sub)licensees of all Promotional Materials that are otherwise related to the Existing Products) and provide a copy of all training materials, medical education materials, packaging and labeling, and all other literature or other information that are related to the Existing Product, which Promotional Materials will be deemed Partner's "Confidential Information" to the extent they also relate to Partner or its Affiliates' other businesses or products and uniQure or its Affiliates and (sub)licensees may use the same to Exploit such Existing Products.

Unless this Agreement is terminated by Partner pursuant to Section 13.2.2 (Termination for Cause), each Party will bear its own costs and expenses associated with the matters described in this Section 13.3.5 (Assignment and Disclosure). If this Agreement is terminated by Partner pursuant to Section 13.2.2 (Termination for Cause), then uniQure will reimburse Partner for the costs and documented out-of-pocket expenses of Partner associated with the assignments set forth in this Section 13.3.5 (Assignment and Disclosure).

To the extent that any agreement or other asset described in this Section 13.3.5 (Assignment and Disclosure) is not assignable by Partner, then such agreement or other asset will not be assigned, and, upon the request of uniQure, Partner will take such reasonable steps as may be necessary to allow uniQure to obtain and to enjoy the benefits of such agreement or other asset, without additional payment therefor, in the form of a license or other right to the extent Partner has the right and ability to do so, and in the event this Agreement is terminated by Partner pursuant to Section 13.2.2 (Termination for Cause), uniQure will reimburse Partner for the costs and documented out-of-pocket expenses incurred by Partner in doing so. For clarity, uniQure will have the right to request that Partner take any or all of the foregoing actions in whole or in part, or with respect to all or any portion of the assets set forth in this Section 13.3.5 (Assignment and Disclosure).

13.3.6 **Regulatory Transfer Support**. In furtherance of the assignment of Regulatory Submissions, Regulatory Approvals, Reimbursement Approvals, and other documentation pursuant to Section 13.3.3 (Regulatory Submissions and Regulatory Approvals) and the other data described in Section 13.3.5 (Assignment and Disclosure), if and to the extent that such assignments have not been made within the [*] after the effective date of termination of this Agreement, Partner will appoint uniQure as Partner's or its Affiliate's agent for all Existing Product-related matters involving Regulatory Authorities until all Regulatory Submissions, Regulatory Approvals, Reimbursement Approvals, and the data described in Section 13.3.5 (Assignment and Disclosure) that are not then in uniQure's or its Affiliate's name have been assigned to uniQure or its designee. In the event of failure to obtain such assignment, Partner hereby consents and grants to uniQure the right to access and reference (without any further action required on the part of Partner, whose authorization to file this consent with any Regulatory Authority is hereby granted) any such item.

13.3.7 **Know-How Transfer Support**. In furtherance of the assignment of Know-How pursuant to Section 13.3.5 (Assignment and Disclosure), Partner will, for a period of [*] from the effective date of termination of this Agreement, provide such consultation or other assistance as uniQure may reasonably request to assist uniQure in becoming familiar with such Know-How in order for uniQure to undertake further Exploitation of the Existing Product, at uniQure's cost and expense; *provided* that if the Agreement is terminated by Partner for any reason other than pursuant to Section 13.2.2 (Termination for Cause), then Partner will provide [*].

13.3.8 **Inventory**. At uniQure's election and request, Partner will transfer to uniQure or its designee some or all inventory of the Existing Product (including all final product, bulk drug substance, intermediates, works-in-process, formulation materials, reference standards, drug product clinical reserve samples, packaged retention samples, and the like) then in the possession or Control of Partner, its Affiliates or Sublicensees; *provided* that uniQure will pay Partner a price equal to [*].

13.3.9 **Wind Down and Transition**. Partner will be responsible, at its own cost and expense (subject to Section 13.4 (Further Effects of Termination by Partner for Cause)), for the wind-down of Partner's and its Affiliates' and its Sublicensees' activities with respect to the Existing Product. Partner will, and will cause its Affiliates and Sublicensees to, reasonably cooperate with uniQure to facilitate orderly transition of all Development, Manufacturing, and Commercialization activities, and other activities, then being performed by or on behalf of Partner or its Affiliates or Sublicensees for the Existing Product to uniQure or its designee, including reasonably cooperating with uniQure to transfer all Development, Manufacturing, and Commercialization activities, and other activities, to uniQure or its designee and continuing to perform such activities on uniQure's behalf for a reasonable time after termination of this Agreement with respect to such Existing Product until such transition is completed.

13.3.10 **Return of Confidential Information**. At the Disclosing Party's election, the Receiving Party will return (at Disclosing Party's expense) or destroy all tangible materials comprising, bearing, or containing any Confidential Information of the Disclosing Party relating to the Existing Product that are in the Receiving Party's or its Affiliates' or Sublicensees' possession or control and provide written certification of such destruction (except to the extent any information is the Confidential Information of both Parties or to the extent that the Receiving Party has the continuing right to use the Confidential Information under this Agreement); *provided* that the Receiving Party may retain one copy of such Confidential Information for its legal archives. Notwithstanding any provision to the contrary set forth in this Agreement, the Receiving Party will not be required to destroy electronic files containing such Confidential Information that are made in the ordinary course of its or its Sublicensees' business information back-up procedures pursuant to its electronic record retention and destruction practices that apply to its or their own general electronic files and information.

13.3.11 **Further Assistance**. Partner will provide any other assistance or take any other actions, in each case, reasonably requested by uniQure, as necessary to transfer to uniQure all Exploitation of the Existing Product, and will execute all documents as may be reasonably requested by uniQure in order to give effect to this Section 13.3 (Effect of Termination other than by Partner for Cause).

13.4 Further Effects of Termination by Partner for Cause. Notwithstanding any provision to the contrary in this Article 13 (Term and Termination), if Partner terminates this Agreement pursuant to Section 13.2.2 (Termination for Cause), then uniQure will bear its

own costs and expenses and will reimburse Partner for the out-of-pocket expenses incurred by Partner and its Affiliates in connection with the matters described in Section 13.3 (Effect of Termination). Partner will invoice uniQure quarterly for the foregoing costs incurred by or on behalf of Partner and its Affiliates in such Financial Quarter, and uniQure will pay the undisputed invoiced amounts within [*] after the date of any such invoice.

13.5 **Survival**. Expiration or termination of this Agreement will not relieve the Parties of any obligation accruing prior to such expiration or termination. Without limiting the foregoing, the following provisions of this Agreement will survive the expiration or termination of this Agreement: Article 1 (Definitions), Section 2.3 (License Grants to uniQure), Section 3.2.3 (Reimbursement of Costs and Expenses) (with respect to amounts becoming due during the Term), Section 3.3.2 (Reimbursement of Costs and Expenses) (with respect to amounts becoming due during the Term), Section 3.5.3 (Cost) (with respect to amounts becoming due during the Term), Section 4.4.4 (Allocation of Costs) (with respect to amounts becoming due during the Term), Section 5.2 (Manufacturing Development Plan) (with respect to amounts becoming due during the Term), Section 5.4 (Transfer of Manufacturing Know-How) (with respect to amounts becoming due during the Term), Section 8.3.3 (Royalty Payments and Reports) (with respect to payments becoming due during the Term), Section 8.4 (Development Payment) (with respect to the Development Payment accrued prior to the Outside Date and due after the termination of this Agreement in accordance with Section 15.5), Section 8.5 (Other Amounts Payable) (with respect to amounts becoming due during the Term), Section 8.9 (Currency; Exchange Rate) (with respect to amounts becoming due during the Term), Section 8.10 (Blocked Payments) (with respect to amounts becoming due during the Term), Section 8.11 (Late Payments) (with respect to amounts becoming due during the Term), Section 8.12 (Financial Records and Audits), Section 9.1 (Duty of Confidence), Section 9.2 (Confidential Information), Section 9.3 (Exemptions), Section 9.4 (Authorized Disclosures), Section 9.6 (Publications), Section 9.7 (Publicity; Use of Names), Section 9.8 (Attorney-Client Privilege), Section 9.9 (Personal Information), Section 10.5 (No Other Warranties), Section 10.8 (Limitation on Claims), Article 11 (Indemnification), Section 12.1.1 (Ownership), Section 12.2 (Practice Under and Other Use of Joint Technology), Section 12.4.3 (Joint Patent Rights), Section 12.6.2 (Defense), Section 12.9 (Cooperation), Section 12.10 (Privilege), Section 13.1 (Term), Section 13.3 (Effect of Termination), Section 13.4 (Further Effects of Termination by Partner for Cause), Section 13.5 (Survival), Section 13.6 (Termination Not Sole Remedy), Article 14 (Dispute Resolution), Section 15.2 (Coordination and Cooperation) (with respect to amounts becoming due during the Term), Section 15.5 (Outside Date) (with respect to amounts becoming due thereunder), and Article 16 (Miscellaneous).

13.6 **Termination Not Sole Remedy**. Termination is not the sole remedy under this Agreement and, whether or not termination is effected and notwithstanding anything to the contrary set forth in this Agreement, all other remedies will remain available except as expressly set forth herein.

ARTICLE 14
DISPUTE RESOLUTION

14.1 **Pre-Arbitration Negotiation.** Except in relation to any arbitration referred to this Article 14 (Dispute Resolution) under Section 8.2.4 (Payment Adjustment for [*]), Section 13.2.2(b)(ii) (CRE Explanation) or Section 13.2.2(c) (Payment Adjustment in Lieu of Termination for Third Party Competitive Product), prior to the commencement of any arbitration hereunder, the Parties, through their respective Executive Officers, will attempt to resolve any dispute for a period of [*].

14.2 **Arbitration**. Except as specifically provided for within this Article 14 (Dispute Resolution), all disputes arising out of or in connection with this Agreement, other than any dispute subject to the provisions of Section 7.5 (Resolution of JSC Disputes), shall be finally settled under the Rules of Arbitration of the International Chamber of Commerce ("**ICC**") by three arbitrators, *provided* that, other than with respect to the arbitration referenced in Section 13.2.3(b) (Termination for Patent Challenge), any dispute as to the scope, validity, enforceability, or infringement of any Patent Rights or trademark rights shall be finally resolved by a court of competent jurisdiction in the country or region in which such Patent Rights or trademark rights were granted or arose.

14.3 **Arbitrator Selection**. The claimant(s) shall nominate one arbitrator in the request for arbitration. The respondent(s) shall nominate one arbitrator in the answer to the request. The two party-nominated arbitrators shall then have [*] to agree, in consultation with the parties to the arbitration, upon the nomination of a third arbitrator to act as president of the tribunal, barring which the ICC Court shall select the third arbitrator (or any arbitrator that claimant(s) or respondent(s) shall fail to nominate in accordance with the foregoing). Each arbitrator shall be experienced in ICC arbitration and in the resolution of disputes concerning research, development and commercialization collaborations in the pharmaceutical industry.

14.4 **Place and Language of Arbitration**. The place of arbitration shall be New York, New York. The language of the arbitration shall be English.

14.5 **Costs of the Arbitration**. The parties to any arbitration conducted pursuant to this Article 14 (Dispute Resolution) shall each bear their own share of the costs of the arbitration and their own attorneys' fees and other advisor, consultant, witness or expert fees and costs.

14.6 **Expedited Arbitration**. In respect of any arbitration commenced under Section 14.2 (Arbitration), any party to such arbitration may elect that the arbitration proceed under the ICC's Expedited Procedure Rules, irrespective of the amount in dispute, in which event such rules shall apply.

14.7 **Specified Expedited Arbitrations**. Any dispute with respect to Milestone Payments or Royalty Rates in connection with the renegotiation thereof under Section 8.2.4 (Payment Adjustment for [*]) or Section 13.2.2(c) (Payment Adjustment in Lieu of Termination for Third Party Competitive Product) or any dispute with respect to the allocation of the Regulatory Milestone Payment relating to the [*] milestone under Section 8.2.5(b)

(Payment Condition and Adjustments for [*] Milestone) shall be subject to arbitration under the ICC's Expedited Procedure Rules as modified herein, irrespective of the amount in dispute, before a sole arbitrator appointed on an expedited basis by the ICC Court. The arbitrator shall be experienced in ICC arbitration and be a professional in business or licensing experienced in the valuation of biopharmaceutical products with at least 10 years of experience in the pharmaceutical and life sciences industries, including the conduct of research, development and commercialization collaborations. Either Party may commence arbitration by notifying the ICC Secretariat of a dispute requiring resolution under this Section 14.7 and requesting the expedited appointment of an arbitrator within a period of no more than [*]. The Parties shall then exchange and provide the arbitrator, within [*] of the arbitrator's appointment, written proposals for (i) in the case of a dispute with respect to Milestone Payments or Royalty Rates in connection with the renegotiation thereof under Section 8.2.4 (Payment Adjustment for [*]) or Section 13.2.2(c) (Payment Adjustment in Lieu of Termination for Third Party Competitive Product), the amount of the Milestone Payments (for the same Milestone Events set forth in Table 8.2.1 in Section 8.2.1 (Regulatory Milestones) and Table 8.2.2 in Section 8.2.2 (Sales Milestones), unless the Parties otherwise expressly agree in writing) or the percentages of the Royalty Rates (for the same annual Net Sales tiers set forth in Table 8.3.1 in Section 8.3.1 (Royalty Payments), unless the Parties otherwise expressly agree in writing) they each propose to be made, or (ii) in case of a dispute with respect to the allocation of the Regulatory Milestone Payment relating to the [*] milestone under Section 8.2.5(b) (Payment Condition and Adjustments for [*] Milestone), the allocation of the Regulatory Milestone Payment relating to the [*] milestone they each propose, in each case together with their reasons therefor and any supporting materials they may wish to submit. In rendering an award, the arbitrator shall be limited to selecting only one of the two proposals submitted by the Parties. The arbitrator shall render an award within [*] of receiving the Parties' written proposals. The arbitrator shall have no jurisdiction to order any form of discovery process or other evidentiary procedures and shall be confined to conducting the process as described above. The arbitrator's award shall be final and binding on the Parties.

14.8 **Injunctive Relief**. Notwithstanding any provision to the contrary set forth in this Agreement, in the event of an actual or threatened breach hereunder, the aggrieved Party may seek equitable relief (including restraining orders, specific performance or other injunctive relief) in any court or other forum, without first submitting to the dispute resolution procedures set forth in Section 14.1 (Pre-Arbitration Negotiation).

14.9 **Confidentiality**. Any and all activities conducted under this Article 14 (Dispute Resolution), including any and all non-public proceedings and decisions under Section 14.2 (Arbitration) or Section 14.7 (Specified Expedited Arbitrations), will be the Confidential Information of each of the Parties, and will be subject to the terms of Article 9 (Confidentiality; Publication).

14.10 **Tolling**. The Parties agree that all applicable statutes of limitation and time-based defenses (such as estoppel and laches) will be tolled once the dispute resolution procedures set forth in this Article 14 (Dispute Resolution) have been initiated and for so long as they are pending.

ARTICLE 15
EFFECTIVENESS

15.1 **Effective Date**. Except for Partner's obligations under Section 8.4 (Development Payment), and the Parties' rights and obligations under Section 2.6 (Exclusivity Covenant), Article 9 (Confidentiality; Publication), Article 10 (Representations, Warranties and Covenants) (except Section 10.7 (Compliance with Laws) and Section 10.8 (Limitation on Claims)) and this Article 15 (Effectiveness), which will be effective as of the Execution Date, this Agreement will not become effective until the first Business Day after the Antitrust Clearance Date (the "**Effective Date**"); *provided* that the Effective Date will not occur (i) if either Party exercises its termination right under Section 15.5 (Outside Date) or (ii) if and for so long as there is in force any Applicable Law enjoining or prohibiting the consummation of the transactions contemplated by this Agreement in the U.S., United Kingdom, or Australia, or an action or proceeding brought by a Governmental Authority is pending that would reasonably be expected to lead to such an injunction or prohibition.

15.2 **Antitrust Filings**. The Parties will use reasonable best efforts to file the Antitrust Filings with the Antitrust Agencies as promptly as reasonably practicable and advisable. [*]

15.3 **Coordination and Cooperation.** Subject to reasonable confidentiality protections, each Party will furnish to the other Party such information and assistance as such other Party may reasonably request in connection with any Antitrust Filings and will reasonably cooperate with the other Party in the preparation and execution of all documents and materials that are to be submitted to the Antitrust Authorities in connection with the Antitrust Filings. Notwithstanding anything herein to the contrary, if the Parties disagree upon any proposed timing, strategy, communication, or activities necessary to obtain any clearance, expiry of applicable waiting period, or waiver of jurisdiction required under any Antitrust Laws for the consummation of this Agreement and the transactions contemplated hereby, the Parties agree to work together in good faith to resolve the disagreement in a mutually acceptable manner. The Parties will, and will instruct their respective counsel to, cooperate with each other and use reasonable best efforts to facilitate and expedite the identification and resolution of any issues arising under any Antitrust Laws at the earliest practicable dates. Such reasonable best efforts and cooperation include counsel's undertaking (a) to keep each other appropriately informed of communications from and to personnel of the reviewing Governmental Authorities, and (b) to confer with each other regarding appropriate contacts with and response to personnel of such Governmental Authorities and the content of any such contacts or presentations. Neither uniQure nor Partner will participate in any meeting or discussion with any Governmental Authority with respect of any Antitrust Filings, investigation, or other inquiry without giving the other party prior notice of the meeting or discussion and, to the extent permitted by the relevant Antitrust Authority, the opportunity to attend and participate in such meeting or discussion (which, at the request of either uniQure or Partner, will be limited to outside antitrust counsel only). Subject to Section 15.2 (Antitrust Filing) and this Section 15.3 (Coordination and Cooperation), uniQure and Partner will each approve the content of any presentations, white papers or other written materials to be submitted to any Governmental Authority with respect to any Antitrust Filing in advance of any such submission; *provided* that (i) information reasonably deemed to be competitively sensitive may be restricted to

outside counsel only, and (ii) information related to valuation of the Licensed Product or any assets of Partner may be redacted.

15.4 **Efforts.** The Parties will each use reasonable best efforts to ensure that any applicable waiting period under the applicable Antitrust Law with respect to the Antitrust Filings expires or is terminated as soon as practicable or to obtain any necessary approvals or consents under such applicable Antitrust Law, at the earliest possible date after the date of the Antitrust Filings; *provided*, that nothing in this Section 15.4 (Efforts) or otherwise will require Partner or any of its Affiliates to, and uniQure and its Affiliates will not without the prior written consent of Partner, take or agree to any action with respect to Partner or any of its Affiliates, or the Licensed Product, including selling, divesting, or otherwise disposing of, holding separate or taking or committing to take any action that limits in any material respect Partner's freedom of action with respect to, or its ability to retain, any business, products, rights, services, assets, or other properties of Partner or any of its Affiliates, or the Licensed Product, or any interest therein. Notwithstanding the foregoing (i) the Parties shall use reasonable best efforts to answer, address, respond to, or rebut any and all substantive concerns, questions, and preliminary objections raised by the U.S. Department of Justice, the FTC, or any other Antitrust Agency that would otherwise prevent, impede or delay the consummation of this Agreement, and (ii) if the U.S. Department of Justice or the FTC has authorized a complaint, another Antitrust Agency has adopted a final decision, or a Court has adopted a final judgment that would prevent, impede or delay the consummation of this Agreement, nothing in this Section 15.4 (Efforts) or otherwise will require Partner or any of its Affiliates to, and uniQure and its Affiliates will not without the prior written consent of Partner, take any action to resolve any such objection, action, suit, or proceeding so as to permit consummation of this Agreement.

15.5 **Outside Date**. Prior to the Effective Date, either Party may terminate this Agreement with immediate effect upon written notice to the other Party (a) if any Antitrust Agency or court of appropriate jurisdiction prohibits, opposes or permanently enjoins the transactions contemplated by this Agreement, or (b) in the event that the Antitrust Clearance Date does not occur on or prior to [*] (the "**Outside Date**"), *provided* that prior to the Outside Date the Parties may agree to extend the Outside Date to a later date (whereupon the "Outside Date" shall be deemed to be such agreed later date). If either Party elects to terminate this Agreement in accordance with the foregoing clause (a) or (b), then no later than [*] after the other Party's receipt of written notice of such termination, Partner will pay to uniQure the Development Payment by wire transfer of immediately available funds to an account specified in writing by uniQure, to the extent the Development Payment has accrued pursuant to Section 8.4 (Development Payment). If (i) the Antitrust Clearance Date does not occur on or prior to the Outside Date (other than as a result of an event specified in foregoing clause (a)), (ii) prior to the Outside Date Partner requests an extension of the Outside Date of no longer than [*], uniQure does not agree to such extension, and this Agreement is terminated pursuant to this Section 15.5 (Outside Date), and (iii) prior to, on or within [*] after the Outside Date, uniQure or any of its Affiliates consummates, enters into any definitive agreement with respect to, or recommends to its shareholders to vote in favor of or to accept an offer with respect to, an Alternative Transaction, then no later than [*] after written demand by Partner, uniQure will pay to Partner, by wire transfer of immediately available funds to an account specified in writing by Partner, the amount equal

to [*]. Such amount will be irrevocable, non-refundable, and non-creditable against any other payment due to Partner pursuant to this Agreement.

Article 16
MISCELLANEOUS

16.1 **Assignment**. Except as otherwise expressly provided herein, this Agreement may not be assigned or otherwise transferred, nor may any right or obligation hereunder be assigned or transferred, by either Party without the prior written consent of the other Party. Notwithstanding any provision to the contrary set forth in this Agreement, (a) uniQure may assign its rights to receive payments under this Agreement to one or more Persons without consent of Partner (including as part of a royalty factoring transaction), and (b) either Party may, without consent of the other Party, assign this Agreement and its rights and obligations hereunder (i), in whole or in part, to an Affiliate of such Party, or (ii) in whole to its successor-in-interest in connection with the sale, transfer, succession, or reorganization of all or substantially all of its assets, whether in a merger, acquisition, or similar transaction or series of related transactions; *provided* that in the case of the foregoing clauses (i) or (ii), the assigning Party provides written notice of such assignment to the non-assigning Party within [*] after the effective date of such assignment, *provided*, *further* that the assignee agrees in writing to assume all of the assigning Party's obligations under this Agreement that are being assigned. If uniQure or any of its Affiliates sells, transfers or exclusively licenses substantially all of the uniQure Technology to any other Person pursuant to the foregoing clause (ii), (x) uniQure shall promptly notify Partner in writing, (y) such Person must assume in writing (and is otherwise deemed to assume automatically) all of uniQure and its Affiliates' obligations herein with respect to such uniQure Technology, and (z) at Partner's request within [*] after such notification, uniQure will cause such Person to execute a direct license to Partner with the same or substantially the same applicable terms herein. Any attempted assignment of this Agreement or other transaction not in accordance with this Section 16.1 (Assignment) will be null, void, and of no legal effect. The terms of this Agreement will be binding upon, and will inure to the benefit of, the Parties and their respected successors and permitted assigns.

16.2 **LIMITATION OF LIABILITY**. NEITHER PARTY WILL BE LIABLE TO THE OTHER FOR ANY SPECIAL, CONSEQUENTIAL, INCIDENTAL, PUNITIVE, OR INDIRECT DAMAGES ARISING FROM OR RELATING TO ANY BREACH OF THIS AGREEMENT, OR DAMAGES FOR LOSS OF PROFIT IN CONNECTION WITH THIS AGREEMENT, IN EACH CASE, REGARDLESS OF ANY NOTICE OF THE POSSIBILITY OF SUCH DAMAGES. NOTWITHSTANDING THE FOREGOING, NOTHING IN THIS SECTION 16.2 (LIMITATION OF LIABILITY) IS INTENDED TO OR WILL LIMIT OR RESTRICT THE INDEMNIFICATION RIGHTS OR OBLIGATIONS OF ANY PARTY UNDER SECTION 11.1 (INDEMNIFICATION; BY PARTNER) OR SECTION 11.2 (INDEMNIFICATION; BY UNIQURE), OR DAMAGES AVAILABLE TO A PARTY FOR THE OTHER PARTY'S (I) MISAPPROPRIATION OR INFRINGEMENT OF INTELLECTUAL PROPERTY OWNED OR CONTROLLED BY SUCH PARTY, (II) BREACH OF ITS OBLIGATIONS UNDER SECTION 2.6 (EXCLUSIVITY COVENANT), (III) BREACH OF THE LICENSES GRANTED TO THE OTHER PARTY UNDER THIS

AGREEMENT, (IV) BREACH OF ITS OBLIGATIONS UNDER ARTICLE 9 (CONFIDENTIALITY; PUBLICATION), OR (V) FRAUD OR WILLFUL MISCONDUCT.

16.3 **Section 365(n) of the Bankruptcy Code**. All rights and licenses granted under or pursuant to this Agreement by a Party to the other, including those set forth in Section 2.1 (License Grants to Partner, Licensed Products and AAV5 NAb Assay) and Section 2.3 (License Grant to uniQure), are and will otherwise be deemed to be, for purposes of Section 365(n) of the U.S. Bankruptcy Code or any foreign counterpart thereto, licenses of right to "intellectual property" as defined under Section 101 of the U.S. Bankruptcy Code or any foreign counterpart thereto. The Parties agree that the Parties will retain and may fully exercise all of their rights and elections under the U.S. Bankruptcy Code and any foreign counterpart thereto. All payments to be made by Partner under this Agreement, including the Upfront Payment, Milestone Payments and Royalties, will be considered "royalties" for purposes of Section 365(n) of the U.S. Bankruptcy Code and any applicable foreign counterpart thereto.

16.4 **Severability**. If any one or more of the provisions contained in this Agreement is held invalid, illegal or unenforceable in any respect, the validity, legality, and enforceability of the remaining provisions contained herein will not in any way be affected or impaired thereby, unless the absence of the invalidated provisions adversely affects the substantive rights of the Parties. The Parties will in such an instance use their best efforts to replace the invalid, illegal, or unenforceable provisions with valid, legal, and enforceable provisions that, insofar as practical, implement the purposes of this Agreement.

16.5 **Notices**. All notices that are required or permitted hereunder will be in writing, will specifically refer to this Agreement, will be addressed to the appropriate Party at the address specified below or such other address as may be specified by such Party in writing in accordance with this Section 16.5 (Notices) (with a courtesy copy sent by email, which will not constitute notice), and will be deemed to have been given for all purposes (a) when received, if hand-delivered, (b) [*] after being dispatched through a reputable courier service, or (c) [*] after mailing, if mailed by first class certified or registered airmail, postage prepaid, return receipt requested.

If to uniQure:

uniQure biopharma B.V.
Paasheuvelweg 25A
1105 BP Amsterdam
The Netherlands
Attention: Chief Accounting Officer

with a copy to (which will not constitute notice):

legalnotices@uniQure.com

and

Ropes & Gray LLP
800 Boylston Street; Prudential Tower
Boston, MA 02199
Attention: David M. McIntosh
Email: David.McIntosh@ropesgray.com

If to Partner:

CSL Behring LLC
1020 First Avenue
King of Prussia, PA 19406
Attention: Group General Counsel
with a copy to (which will not constitute notice):

Simpson Thacher & Bartlett LLP
425 Lexington Avenue
New York, NY 10017
Attention: Mark D. Pflug and Lori Lesser
Email: mpflug@stblaw.com; llesser@stblaw.com

16.6 **Governing Law**. This Agreement, and all claims or causes of action (whether in contract, tort or statute) that may be based upon, arise out of or relate to this Agreement, or the negotiation, execution or performance of this Agreement or the breach thereof (including any claim or cause of action based upon, arising out of or related to any representation or warranty made in or in connection with this Agreement or as an inducement to enter into this Agreement), will be governed by, and enforced in accordance with, the internal laws of the State of New York, including its statutes of limitations without giving effect to the conflicts of law provisions thereunder.

16.7 **Force Majeure**. Neither Party will be held liable to the other Party nor be deemed to have defaulted under or breached this Agreement for failure or delay in achieving any objective, satisfying any condition, or performing any obligation under this Agreement to the extent that such failure or delay is caused by or results from acts or events beyond the reasonable control of such Party, including acts of God, embargoes, war, acts of war (whether war be declared or not), terrorism, insurrections, riots, civil commotions, strikes, lockouts, or other labor disturbances (other than strikes, lockouts, or labor disturbances involving a Party's own employees), government actions, fire, earthquakes, floods, epidemics, pandemics, the spread of infectious diseases, quarantines, and the COVID-19 pandemic (notwithstanding that the COVID-19 pandemic and related government orders are ongoing as of the Execution Date, so long as its effects are not reasonably foreseeable as of the Execution Date) ("**Force Majeure**"). The affected Party will notify the other Party in writing of any Force Majeure circumstances that may affect its performance under this Agreement as soon as reasonably practicable, will provide a good faith estimate of the period for which its failure or delay in performance under the Agreement is expected to continue based on currently available information, and will undertake reasonable efforts necessary to mitigate and overcome such Force Majeure circumstances and resume normal performance of its

obligations hereunder promptly under the circumstances. If the Force Majeure circumstance continues, then the affected Party will update such written notice to the other Party on a bi-weekly basis, or more frequently if requested by the other Party, to provide updated summaries of its mitigation efforts and its estimates of when normal performance under this Agreement will be able to resume.

16.8 **Entire Agreement; Amendments**. This Agreement, together with the Schedules hereto and the other agreements executed by the Parties and their respective Affiliates in connection herewith (including the Supply Agreement), contains the entire understanding of the Parties with respect to the subject matter hereof. Any other express or implied agreements and understandings, negotiations, writings and commitments, either oral or written, in respect to the subject matter hereof (including the collaboration and the licenses granted hereunder) are superseded by the terms of this Agreement. The Schedules to this Agreement are incorporated herein by reference and will be deemed a part of this Agreement. This Agreement may be amended, or any term hereof modified, only by a written instrument duly executed by authorized representatives of each Party. The foregoing will not be interpreted as a waiver of any remedies available to either Party or its Affiliates as a result of any breach, prior to the Effective Date, by the other Party or its Affiliates of such Party's or its Affiliate's obligations pursuant to the Confidential Disclosure Agreement.

16.9 **Headings**. The captions to the several Articles, Sections, and subsections hereof are not a part of this Agreement but are merely for convenience to assist in locating and reading the several Articles, Sections and subsections of this Agreement.

16.10 **Independent Contractors**. It is expressly agreed that uniQure and Partner will be independent contractors and that the relationship between the two Parties will not constitute a partnership, joint venture or agency. Neither uniQure nor Partner will have the authority to make any statements, representations, or commitments of any kind, or to take any action that is binding on the other Party without the prior written consent of the other Party.

16.11 **Performance by Affiliates**. Notwithstanding any provision to the contrary set forth in this Agreement, each Party will have the right to perform any or all of its obligations and exercise any or all of its rights under this Agreement through any Affiliate, *provided* that such Party will be and remain responsible and liable for the performance of such obligations. Each Party hereby guarantees the performance by any Affiliates of such Party's obligations under this Agreement and will cause any such performing Affiliates to comply with the provisions of this Agreement in connection with such performance.

16.12 **Waiver**. Any waiver of any provision of this Agreement will be effective only if in writing and signed by uniQure and Partner. No express or implied waiver by a Party of any default under this Agreement will be a waiver of a future or subsequent default. The failure or delay of any Party in exercising any rights under this Agreement will not constitute a waiver of any such right, and any single or partial exercise of any particular right by any Party will not exhaust the same or constitute a waiver of any other right provided in this Agreement.

16.13 **Waiver of Rule of Construction**. Each Party has had the opportunity to consult with counsel in connection with the review, drafting and negotiation of this Agreement. Accordingly, the rule of construction that any ambiguity in this Agreement will be construed against the drafting Party will not apply.

16.14 **Cumulative Remedies**. No remedy referred to in this Agreement is intended to be exclusive, but each will be cumulative and in addition to any other remedy referred to in this Agreement or otherwise available under Applicable Law.

16.15 **Business Day Requirements**. If any notice or other action or omission is required to be taken by a Party under this Agreement on a day that is not a Business Day, then such notice or other action or omission will be deemed to be required to be taken on the next occurring Business Day.

16.16 **Further Actions**. Each Party agrees to execute, acknowledge, and deliver such further instruments, and to do all such other acts, as reasonably necessary in order to carry out the purposes and intent of this Agreement.

16.17 **Construction**. Except where the context expressly requires otherwise, (a) the use of any gender herein will be deemed to encompass references to either or both genders, and the use of the singular will be deemed to include the plural (and vice versa), (b) the words "include," "includes," and "including" will be deemed to be followed by the phrase "without limitation," (c) the word "will" will be construed to have the same meaning and effect as the word "shall," (d) any definition of or reference to any agreement, instrument, or other document herein will be construed as referring to such agreement, instrument, or other document as from time to time amended, supplemented, or otherwise modified (subject to any restrictions on such amendments, supplements or modifications set forth herein), (e) any reference herein to any Person will be construed to include the Person's successors and assigns, (f) the words "herein," "hereof," and "hereunder" and words of similar import, will each be construed to refer to this Agreement in its entirety and not to any particular provision hereof, (g) all references herein to Articles, Sections, Schedules, or Exhibits will be construed to refer to Articles, Sections, Schedules, or Exhibits of this Agreement, and references to this Agreement include all Schedules hereto, (h) the word "notice" means notice in writing (whether or not specifically stated) and will include notices, consents, approvals and other written communications contemplated under this Agreement, (i) provisions that require that a Party, the Parties or any committee hereunder "agree," "consent," "approve," or the like will require that such agreement, consent, or approval be specific and in writing, whether by written agreement, letter, approved minutes, or otherwise (but excluding e-mail and instant messaging), and may be withheld in a Party's sole discretion, unless otherwise specified herein, (j) references to any specific law, rule or regulation, or section or other division thereof, will be deemed to include the then-current amendments thereto or any replacement or successor law, rule or regulation thereof, and (k) the term "or" will be interpreted in the inclusive sense commonly associated with the term "and/or."

16.18 **Language; Translations**. This Agreement is in the English language only, which language will be controlling in all respects, and all versions hereof in any other language

will be for accommodation only and will not be binding upon the Parties. All communications and notices to be made or given by one Party to the other pursuant to this Agreement, and any dispute proceeding related to or arising hereunder, will be in the English language. If there is a discrepancy between any translation of this Agreement and any non-English translation of this Agreement, this Agreement will prevail.

16.19 **Counterparts**. This Agreement may be executed in any number of counterparts by facsimile or PDF signature pages, each of which will be deemed an original, but all of which together will constitute one and the same instrument.

[Remainder of the Page Intentionally Left Blank; Signature Page Follows]

IN WITNESS WHEREOF, the Parties intending to be bound have caused this Commercialization and License Agreement to be executed by their respective duly authorized representatives as of the Execution Date.

uniQure biopharma BV

By: _____

Name: _____

Title: _____

CSL Behring LLC

By: _____

Name: _____

Title: _____

By: _____

Name: _____

Title: _____

Schedule 1.98

uniQure Knowledge Individuals and Partner Knowledge Individuals

(a) uniQure Knowledge Individuals

[*]

(b) Partner Knowledge Individuals

[*]

Schedule 2.2.1

Specified CMOs

[*]

Schedule 2.5

Existing In-Licenses

[*]

Schedule 4.4.1

uniQure Development Budget

[*]

Schedule 5.2

Manufacturing Development Plan

[*]

[*] **[REDACTED]**

[*] **[REDACTED]**

Schedule 9.7.1

Press Releases

[Attached.]

uniQure Announces License Agreement with CSL Behring to Commercialize Hemophilia B Gene Therapy

~ CSL Behring Obtains Exclusive Global Rights to Develop and Commercialize uniQure's Differentiated Gene Therapy Candidate for Hemophilia B ~

~ uniQure to Receive $450 Million in Upfront Cash, Up to an Additional $1.6 Billion in Milestone Payments, and Double-Digit Royalties Ranging Up to a Low-Twenties Percent of Net Sales ~

~ Agreement Leverages CSL Behring's Global Hematology Capabilities and Infrastructure to Benefit Hemophilia B Patients Worldwide ~

~ Transaction Expected to Enable uniQure to Strategically Expand and Accelerate Pipeline and Platform ~

~ uniQure to Host Conference Call Today, June 24, 2020, at 5:30 p.m. EDT ~

Lexington, MA and Amsterdam, the Netherlands, June 24, 2020 — uniQure N.V. (NASDAQ: QURE), a leading gene therapy company advancing transformative therapies for patients with severe medical needs, today announced that uniQure and CSL Behring have entered into a licensing agreement providing CSL Behring exclusive global rights to etranacogene dezaparvovec, uniQure's investigational gene therapy for patients with hemophilia B. Etranacogene dezaparvovec consists of an AAV5 viral vector carrying a gene cassette with the patent-protected Padua variant of Factor IX (FIX-Padua). Under the terms of the agreement, uniQure will receive a $450 million upfront cash payment and be eligible to receive up to $1.6 billion in payments based on regulatory and commercial milestones. uniQure will also be eligible to receive tiered double-digit royalties in a range of up to a low-twenties percent of on net sales of products arising from the collaboration based on sales thresholds.

The collaboration combines uniQure's differentiated gene therapy candidate in hemophilia B and CSL Behring's strong global reach and commercial infrastructure in hematology to accelerate access of etranacogene dezaparvovec to hemophilia B patients around the world.

"We are thrilled to enter into this commercialization and license agreement with CSL Behring, an ideal commercial partner with global reach and decades of expertise in hemophilia," stated Matt Kapusta, chief executive officer of uniQure. "We believe that through this arrangement, we are ideally positioned to deliver our innovative gene therapy to the largest number of hemophilia B patients as quickly as possible."

"The transaction represents a major milestone in the development of etranacogene dezaparvovec and, when closed, we expect that it will provide uniQure with significant financial resources to advance and expand our pipeline of gene therapy candidates, anchored by AMT-130 in Huntington's disease, as well as to invest further in our leading manufacturing and technology platform," he added.

As a CSL Limited (ASX:CSL;USOTC:CSLLY) company, CSL Behring is a global biotherapeutics leader delivering lifesaving medicines to patients with rare and serious diseases. A global leader in treating bleeding disorders, CSL Behring has been delivering innovations for the hemophilia patient community for more than 30 years. The company reported more than $1 billion in sales of hemophilia-related medicines in 2019.

"Our vision with hemophilia B patients is to offer transformational treatment paradigms that help free them from the lifelong burden of this disease," said CSL's CEO and Managing Director Paul Perreault. "With more than three decades of providing lifesaving innovations for the global bleeding disorders community, we are well positioned to maximize the potential benefit of this therapy. Upon approval, we believe this next-generation therapy will be highly complementary to our existing best-in-class hemophilia B product portfolio with an alternate best-in-class treatment option."

Under the terms of the agreement, uniQure will be responsible for the completion of the HOPE-B pivotal study, manufacturing process validation, and the manufacturing supply of etranacogene dezaparvovec until such time that these capabilities are transferred to CSL Behring. Clinical development and regulatory activities performed by uniQure under the agreement will be reimbursed by CSL Behring. CSL Behring will be responsible for regulatory submissions and commercialization of etranacogene dezaparvovec.

The closing of the transaction is contingent on completion of review under antitrust laws in the United States, Australia and the United Kingdom.

Accelerate Build-out of Innovative Gene Therapy Pipeline and Platform

uniQure expects that the agreement will provide significant additional capital to accelerate and expand its pipeline of innovative gene therapies, including advancing the Phase I/II study of AMT-130 in Huntington's disease, initiating IND-enabling studies of AMT-150 in spinocerebellar ataxia type 3, and progressing current and additional candidates for central nervous system disorders and other genetic diseases. uniQure anticipates announcing early safety data from the Phase I/II study of AMT-130 in the second half of 2020 and initial efficacy data in 2021.

uniQure plans to continue to leverage its deep expertise with AAV5 to develop potentially best-in-class gene therapies. AAV5-based gene therapies have been demonstrated to be safe and well tolerated in a multitude of clinical trials, including uniQure trials conducted in hemophilia B and other indications. No patient treated in clinical trials with uniQure's AAV5 gene therapies has experienced any cytotoxic T-cell-mediated immune response to the capsid. Additionally, preclinical and clinical data show that AAV5-based gene therapies may be viable treatments in patients with pre-existing antibodies to AAV5, thereby potentially increasing patient eligibility for treatment. uniQure also may seek to in-license or acquire additional product candidates that align with this research and development strategy.

In addition, uniQure plans to further strengthen its proprietary gene therapy platform by expanding its manufacturing capacity to support a broad pipeline, including product candidates for diseases with larger prevalence, as well as investing further in enabling technologies to improve the efficacy and safety of its gene therapies.

As part of uniQure's effort to focus on those gene therapy programs that have the greatest potential to improve patients' lives and generate long-term value for shareholders, uniQure plans to de-prioritize its research program of AMT-180 for patients with hemophilia A.

Moelis & Company acted as a financial advisor to uniQure in this transaction.

Conference Call Today at 5:30 p.m. EDT

uniQure will host a conference call today, June 24, 2020, at 5:30 p.m. Eastern Daylight Time. The conference call may be accessed by dialing (877) 870-9135 for domestic callers and +44 020 719 283 38 for international callers.

The passcode for the call is 9499239. Please specify to the operator that you would like to join the "uniQure Conference Call." The conference call will be webcast live under the investor relations section of uniQure's website at www.uniqure.com and will be archived there following the call for 90 days.

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 gene therapies to treat patients with hemophilia B, Huntington's disease, Fabry disease, spinocerebellar ataxia Type 3 and other diseases. www.uniQure.com

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, whether the parties will successfully complete the review under applicable antitrust laws or otherwise close the transaction, whether uniQure will receive the upfront cash payment or any of the financial benefits of the agreement; whether the collaboration will benefit Hemophilia B patients worldwide, whether the parties to the agreement will establish a new standard of care for patients with hemophilia B, whether uniQure will be able to accelerate or expand its pipeline of innovative gene therapies or its technology platform, including advancing the Phase I/II study of AMT-130 in Huntington's disease, initiating IND-enabling studies of AMT-150 in spinocerebellar ataxia type 3, or progressing current or additional candidates for central nervous system disorders and other genetic diseases, whether uniQure will announce early safety data from its Phase I/II study of AMT-130 in the second half of 2020 and initial efficacy data in 2021 or ever, whether uniQure will develop best-in-class gene therapies, whether uniQure will in-license or acquire additional product candidates, whether uniQure will expand its manufacturing capacity to support a broad pipeline, such as product candidates for diseases with larger prevalence, and whether uniQure will obtain enabling technologies that improve the efficacy or safety of its gene therapies. uniQure's actual results could differ materially from those anticipated in these forward-looking statements for many reasons, including, without limitation, risks associated with the impact of the ongoing COVID-19 pandemic on our Company and the wider economy and health care system, our clinical development activities, clinical results, collaboration arrangements, 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 Annual Report on Form 10-Q filed on April 29, 2020. Given these risks, uncertainties and other factors, you should not place undue reliance on these forward- looking statements, and uniQure assumes no obligation to update these forward-looking statements, even if new information becomes available in the future.

uniQure Contacts:

FOR INVESTORS:

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Mobile: 617-680-9452
m.cantor@uniQure.com

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t.malone@uniQure.com

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Victoria 3052 Australia

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F +613 9389 1434
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CSL ASX Announcement

For immediate release
25 June 2020

CSL Agrees to Acquire Late-Stage

Gene Therapy Candidate for Haemophilia B from uniQure

CSL Limited (ASX:CSL; USOTC:CSLLY) today announced that CSL has agreed to acquire from uniQure exclusive global license rights to commercialise an adeno- associated virus (AAV) gene therapy program, AMT-061 (etranacogene dezaparvovec), for the treatment of haemophilia B. The AMT-061 program, currently in Phase 3 clinical trials, could be one of the first gene therapies to provide potentially long-term benefits to patients with haemophilia B.

One dose of AMT-061 has shown to increase Factor IX (FIX) plasma levels – the blood clotting protein lacking in people with haemophilia B – to a degree that reduces or eliminates the tendency for bleeding for many years. Should AMT-061 be successful, appropriate candidate haemophilia B patients would be able to have a one-time treatment to restore FIX activity to functional levels capable of eliminating the need for frequent and ongoing replacement therapies.

"Our vision for haemophilia B patients is to offer transformational treatment paradigms that help free them from the lifelong burden of this disease," said CSL's CEO and Managing Director Paul Perreault. "With more than three decades of providing lifesaving innovations for the global bleeding disorders community, we are well positioned to maximise the potential benefit of this therapy."

Under the agreement with uniQure, upon closing the transaction CSL will have the exclusive global right to commercialise AMT-061. uniQure (NASDAQ: QURE), a leading gene therapy company, will receive an upfront cash payment of US$450 million followed by regulatory and commercial sales milestone payments and royalties. Under the terms of the agreement, uniQure will complete the Phase 3 trial and scale up manufacture for early commercial supply under an agreed plan with CSL.

The transaction is subject to customary regulatory clearances before closing.

Mr. Perreault added, "Upon approval, we believe this next-generation therapy would be highly complementary to our existing haemophilia B product portfolio. We hope that it provides patients with an alternate best-in-class treatment option, building on our legacy of delivering lifesaving innovations in hematology."

This acquisition will also enhance CSL's capabilities in its growing gene therapy portfolio. The company is currently developing a stem cell gene therapy (CSL200) for the treatment of sickle cell disease and has recently established an alliance with Seattle Children's Research Institute to develop a stem cell gene therapy for primary immunodeficiency diseases.

About Etranacogene Dezaparvovec (AMT-061)

Etranacogene dezaparvovec, also known as AMT-061, consists of an AAV5 viral vector carrying a gene cassette with the patent-protected Padua variant of Factor IX (FIX- Padua). AAV5-based gene therapies have been demonstrated to be safe and well tolerated in many clinical trials, including four uniQure trials conducted in 25 patients in hemophilia B and other indications. No patient treated in clinical trials with uniQure's AAV5-based gene therapies has experienced any cytotoxic T-cell-mediated immune response to the capsid. Additionally, preclinical and clinical data show that AAV5-based gene therapies may be clinically effective in patients with pre-existing antibodies

to AAV5, thereby potentially increasing patient eligibility for treatment compared to other gene therapy product candidates.

Authorised by
Fiona Mead
Company Secretary

FURTHER INFORMATION

For further information, please contact:

Investors: Media:

Mark Dehring Jemimah Brennan

CSL Limited ABN 99 051 588 348

CSL ASX Announcement

Head of Investor Relations Head of Communications Asia Pacific

CSL Limited CSL Limited

Telephone: +613 9389 3407 Mobile +61 412 635 483

Email: mark.dehring@csl.com.au Email: jemimah.brennan@csl.com.au

CSL Behring NEWS RELEASE

For Immediate Release

CSL Behring Agrees to Acquire Novel Late-Stage Gene Therapy Candidate for Hemophilia B Patients from uniQure

- *Unique gene therapy has the potential to be one of the first to market treatments to provide potentially long- term benefits with only one dose*
- *CSL Behring builds on legacy of delivering innovative treatment options for people with Hemophilia B*

KING OF PRUSSIA, Pa., – 23 June 2020 – Global biotherapeutics leader CSL Behring announced today that it has agreed to acquire exclusive global license rights to commercialize an adeno-associated virus (AAV) gene therapy program, AMT-061 (etranacogene dezaparvovec), for the treatment of hemophilia B from uniQure (NASDAQ: QURE), a leading gene therapy company. The AMT-061 program, currently in Phase 3 clinical trials, could be one of the first gene therapies to provide potentially long-term benefits to patients with hemophilia B.

One dose of AMT-061 has shown to increase Factor IX (FIX) plasma levels – the blood clotting protein lacking in people with hemophilia B – to a degree that reduces or eliminates the tendency for bleeding for many years. Should AMT-061 be successful, appropriate candidate hemophilia B patients would be able to have a one-time treatment to restore FIX activity to functional levels capable of eliminating the need for frequent and ongoing replacement therapies.

"Our vision with hemophilia B patients is to offer transformational treatment paradigms that help free them from the lifelong burden of this disease," said CSL's CEO and Managing Director Paul Perreault. "With more than three decades of providing lifesaving innovations for the global bleeding disorders community, we are well positioned to maximize the potential benefit of this therapy."

Under the agreement with uniQure, upon closing the transaction CSL Behring will have the exclusive global right to commercialize AMT-061. uniQure will receive an upfront cash payment of US$450 million followed by regulatory and commercial sales milestone payments and royalties. Under the terms of the agreement, uniQure will complete the Phase 3 trial and scale up manufacture for early commercial supply under an agreed plan with CSL Behring. The transaction is subject to customary regulatory clearances before closing.

"We are thrilled to enter into this commercialization and license agreement with CSL Behring, an ideal commercial partner with global reach and decades of expertise in hemophilia," stated Matt Kapusta, chief

executive officer of uniQure. “We believe that through this arrangement, we are ideally positioned to deliver our innovative gene therapy to the largest number of hemophilia B patients as quickly as possible. The transaction represents a major milestone in the development of etranacogene dezaparvovec and, when closed, we expect that it will provide uniQure with significant financial resources to advance and expand our pipeline of gene therapy candidates, anchored by AMT-130 in Huntington’s disease, as well as to invest further in our leading manufacturing and technology platform.”

In December 2019, uniQure announced that data from its Phase 2b dose-confirmation study of AMT-061 showed that all patients stabilized and sustained FIX activity at functionally high levels one year after a single dose – with increases in FIX activity of up to 50% of normal and a mean of 41%. This exceeds the levels considered sufficient to eliminate or significantly reduce the risk of bleeding events.

According to CSL Behring’s Executive Vice President and Head of Research and Development Bill Mezzanotte, “We are exceedingly encouraged by the data we’ve seen on AMT-061. Not only has the treatment option demonstrated robust clinically meaningful responses in FIX activity, but it has also exhibited excellent safety over multiple years of observation. Expanding our gene therapy portfolio to treat hemophilia B, a disease state well known to CSL Behring, exemplifies how we are strategically aligning our rare and serious disease focus and our targeted therapeutic area focus with our core scientific platforms to transform the lives of patients.”

This acquisition will also enhance CSL Behring’s capabilities in its growing gene therapy portfolio. The company is currently developing a stem cell gene therapy (CSL200) for the treatment of sickle cell disease and has recently established an alliance with Seattle Children’s Research Institute to develop a stem cell gene therapy for primary immunodeficiency diseases -- another rare disease area where CSL Behring has leading capabilities.

Perreault added, “Upon approval, this next-generation therapy would be highly complementary to our existing best-in-class hemophilia B product portfolio with an alternate best-in-class treatment option. With the license to AMT-061, we are building on our legacy of delivering lifesaving innovations in hematology where, today, we offer a market leading product for hemophilia B and we are a leader in therapies for treating hemophilia A, von Willebrand disease, thrombosis, and other life-threatening conditions.”

CSL Behring has put patients first by addressing the world’s most serious, complicated and rare diseases for over 100 years. The company is now bringing that same commitment to gene therapy; its mission is to address unmet patient needs and enable patients to get the very most out of life.

About Etranacogene Dezaparvovec (AMT-061)

Etranacogene dezaparvovec, also known as AMT-061, consists of an AAV5 viral vector carrying a gene cassette with the patent-protected Padua variant of Factor IX (FIX-Padua). AAV5-based gene therapies have been demonstrated to be safe and well tolerated in many clinical trials, including four uniQure trials conducted in 25 patients in hemophilia B and other indications. No patient treated in clinical trials with uniQure's AAV5-based gene therapies has experienced any cytotoxic T-cell-mediated immune response to the capsid. Additionally, preclinical and clinical data show that AAV5-based gene therapies may be clinically effective in patients with pre-existing antibodies to AAV5, thereby potentially increasing patient eligibility for treatment compared to other gene therapy product candidates.

About CSL Behring

CSL Behring is a global biotherapeutics leader driven by its promise to save lives. Focused on serving patients' needs by using the latest technologies, we develop and deliver innovative therapies that are used to treat coagulation disorders, primary immune deficiencies, hereditary angioedema, respiratory disease, and neurological disorders. The company's products are also used in cardiac surgery, burn treatment and to prevent hemolytic disease of the newborn.

CSL Behring operates one of the world's largest plasma collection networks, CSL Plasma. The parent company, CSL Limited (ASX:CSL;USOTC:CSLLY), headquartered in Melbourne, Australia, employs more than 26,000 people, and delivers its life-saving therapies to people in more than 70 countries. For inspiring stories about the promise of biotechnology, visit Vita CSLBehring.com/vita and follow us on Twitter.com/CSLBehring.

# # #

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Investors:
Mark Dehring

Head of Investor Relations CSL Limited
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Schedule 10.2.1

Permitted Encumbrances

LOAN AND SECURITY AGREEMENT as amended and restated, dated as of May 6, 2016 and December 6, 2018 by and among uniQure biopharma B.V., uniQure, Inc., uniQure IP B.V., uniQure N.V., and Hercules Capital, Inc.

Schedule 10.2.2

uniQure Patent Rights

uniQure Product Patent Rights

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Schedule 10.2.5

Third Party Claims

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Schedule 10.6.2

uniQure Interim Development and Manufacturing Development Plan

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Exhibit 31.1

Certification of Chief Executive Officer

I, Matthew Kapusta, certify that:

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

2. Based on my knowledge, this report does not contain any untrue statement of a material fact or omit to state a material fact necessary to make the statements made, in light of the circumstances under which such statements were made, not misleading with respect to the period covered by this report;

3. Based on my knowledge, the financial statements, and other financial information included in this report, fairly present in all material respects the financial condition, results of operations and cash flows of the registrant as of, and for, the periods presented in this report;

4. The registrant's other certifying officer and I are responsible for establishing and maintaining disclosure controls and procedures (as defined in Exchange Act Rules 13a-15(e) and 15d-15(e)) and internal control over financial reporting (as defined in Exchange Act Rules 13a-15(f) and 15d-15(f)) for the registrant and have:

(a) Designed such disclosure controls and procedures, or caused such disclosure controls and procedures to be designed under our supervision, to ensure that material information relating to the registrant, including its consolidated subsidiaries, is made known to us by others within those entities, particularly during the period in which this report is being prepared;

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

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

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

5. The registrant's other certifying officer and I have disclosed, based on our most recent evaluation of internal control over financial reporting, to the registrant's auditors and the audit committee of the registrant's board of directors (or persons performing the equivalent functions):

(a) All significant deficiencies and material weaknesses in the design or operation of internal control over financial reporting which are reasonably likely to adversely affect the registrant's ability to record, process, summarize and report financial information; and

(b) Any fraud, whether or not material, that involves management or other employees who have a significant role in the registrant's internal control over financial reporting.

By: /s/ MATTHEW KAPUSTA

Matthew Kapusta
Chief Executive Officer
July 30, 2020

Exhibit 31.2

Certification of Chief Financial Officer

I, Matthew Kapusta, certify that:

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

2. Based on my knowledge, this report does not contain any untrue statement of a material fact or omit to state a material fact necessary to make the statements made, in light of the circumstances under which such statements were made, not misleading with respect to the period covered by this report;

3. Based on my knowledge, the financial statements, and other financial information included in this report, fairly present in all material respects the financial condition, results of operations and cash flows of the registrant as of, and for, the periods presented in this report;

4. The registrant's other certifying officer and I are responsible for establishing and maintaining disclosure controls and procedures (as defined in Exchange Act Rules 13a-15(e) and 15d-15(e)) and internal control over financial reporting (as defined in Exchange Act Rules 13a-15(f) and 15d-15(f)) for the registrant and have:

(a) Designed such disclosure controls and procedures, or caused such disclosure controls and procedures to be designed under our supervision, to ensure that material information relating to the registrant, including its consolidated subsidiaries, is made known to us by others within those entities, particularly during the period in which this report is being prepared;

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

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

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

5. The registrant's other certifying officer and I have disclosed, based on our most recent evaluation of internal control over financial reporting, to the registrant's auditors and the audit committee of the registrant's board of directors (or persons performing the equivalent functions):

(a) All significant deficiencies and material weaknesses in the design or operation of internal control over financial reporting which are reasonably likely to adversely affect the registrant's ability to record, process, summarize and report financial information; and

(b) Any fraud, whether or not material, that involves management or other employees who have a significant role in the registrant's internal control over financial reporting.

By: /s/ MATTHEW KAPUSTA

Matthew Kapusta
Principal Financial Officer
July 30, 2020

Exhibit 32.1

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

In connection with this Quarterly Report of uniQure N.V. (the "Company") on Form 10-Q for the period ended June 30, 2020, as filed with the Securities and Exchange Commission on the date hereof (the "Report"), the undersigned, Matthew Kapusta, Chief Executive Officer and Principal 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
Principal Financial Officer
July 30, 2020

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.