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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, 2023

OR

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

For the transition period from _____ to _____

Commission file number: 001-36294

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

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

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

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

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, par value €0.05	QURE	The Nasdaq Stock Market LLC (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 27, 2023, the registrant had 47,703,982 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, include, but are not limited to, statements related to our collaboration, royalty financing and license agreements, our cash runway, the advancement of our clinical trials, and the impact of regulatory actions on our regulatory submission and approval timelines.

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 (the "SEC"), including our most recent Annual Report on Form 10-K filed with the SEC on February 27, 2023 (the "Annual Report"), 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, 2023, and in our Annual Report, including in "Part I, Item 1A. Risk Factors," as well as others that we may consider immaterial or do not anticipate at this time. These cautionary statements should be considered in connection with any written or oral forward-looking statements that we may make in the future or may file or furnish with the SEC. We do not undertake any obligation to release publicly any revisions to these forward-looking statements after completion of the filing of this Quarterly Report on Form 10-Q to reflect later events or circumstances or to reflect the occurrence of unanticipated events. All forward-looking statements attributable to us are expressly qualified in their entirety by these cautionary statements.

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

Part I – FINANCIAL INFORMATION

Item 1. Financial Statements

uniQure N.V.

UNAUDITED CONSOLIDATED BALANCE SHEETS

	June 30, 2023	December 31, 2022
	(in thousands, except share and per share amounts)	
Current assets		
Cash and cash equivalents	$ 513,598	$ 228,012
Current investment securities	114,989	124,831
Accounts receivable and contract asset	102,559	102,376
Inventories	10,212	6,924
Prepaid expenses	12,351	11,817
Other current assets and receivables	3,250	2,814
Total current assets	**756,959**	**476,774**
Non-current assets		
Property, plant and equipment, net of accumulated depreciation of $49.4 million as of June 30, 2023 and $44.1 million as of December 31, 2022	48,567	50,532
Non-current investment securities	—	39,984
Operating lease right-of-use assets	31,395	32,726
Intangible assets, net, including in-process research and development asset of $58.3 million as of June 30, 2023 and $57.3 million as of December 31, 2022	59,713	58,778
Goodwill	26,016	25,581
Deferred tax assets, net	13,995	14,528
Other non-current assets	6,124	6,061
Total non-current assets	**185,810**	**228,190**
Total assets	**$ 942,769**	**$ 704,964**
Current liabilities		
Accounts payable	$ 9,373	$ 10,984
Accrued expenses and other current liabilities	23,537	30,571
Current portion of contingent consideration	27,666	25,982
Current portion of operating lease liabilities	7,780	8,382
Total current liabilities	**68,356**	**75,919**
Non-current liabilities		
Long-term debt	101,110	102,791
Liability from royalty financing agreement	372,445	—
Operating lease liabilities, net of current portion	30,195	31,719
Contingent consideration, net of current portion	9,581	9,334
Deferred tax liability, net	6,802	8,257
Other non-current liabilities	960	935
Total non-current liabilities	**521,093**	**153,036**
Total liabilities	**589,449**	**228,955**
Commitments and contingencies		
Shareholders' equity		
Ordinary shares, €0.05 par value: 80,000,000 shares authorized as of June 30, 2023 and December 31, 2022 and 47,702,331 and 46,968,032 ordinary shares issued and outstanding as of June 30, 2023 and December 31, 2022, respectively	2,877	2,838
Additional paid-in-capital	1,130,515	1,113,393
Accumulated other comprehensive loss	(52,440)	(58,291)
Accumulated deficit	(727,632)	(581,931)
Total shareholders' equity	**353,320**	**476,009**
Total liabilities and shareholders' equity	**$ 942,769**	**$ 704,964**

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,	
	2023	2022	2023	2022
	(in thousands, except share and per share amounts)		(in thousands, except share and per share amounts)	
License revenues	$ 793	$ —	$ 793	$ —
Contract manufacturing revenues	1,310	—	$ 6,247	$ —
Collaboration revenues	319	497	707	2,289
Total revenues	**2,422**	**497**	**7,747**	**2,289**
Operating expenses:				
Cost of contract manufacturing revenues	(1,352)	(832)	(3,787)	(832)
Research and development expenses	(46,036)	(46,192)	(106,845)	(91,195)
Selling, general and administrative expenses	(21,181)	(12,491)	(39,029)	(23,478)
Total operating expenses	**(68,569)**	**(59,515)**	**(149,661)**	**(115,505)**
Other income	1,302	3,186	3,113	3,496
Other expense	(229)	(229)	(445)	(422)
Loss from operations	**(65,074)**	**(56,061)**	**(139,246)**	**(110,142)**
Interest income	3,229	35	4,898	78
Interest expense	(6,840)	(2,694)	(10,402)	(5,210)
Foreign currency gains / (losses), net	374	19,398	(1,995)	27,966
Other non-operating gains, net	—	(57)	—	635
Loss before income tax benefit	**$ (68,311)**	**$ (39,379)**	**$ (146,745)**	**$ (86,673)**
Income tax (expense)/ benefit	(163)	318	1,044	934
Net loss	**$ (68,474)**	**$ (39,061)**	**$ (145,701)**	**$ (85,739)**
Other comprehensive loss:				
Foreign currency translation adjustments	54	(28,324)	5,851	(38,774)
Total comprehensive loss	**$ (68,420)**	**$ (67,385)**	**$ (139,850)**	**$ (124,513)**
Basic and diluted net loss per ordinary share	(1.44)	(0.84)	(3.06)	(1.84)
Weighted average shares used in computing basic and diluted net loss per ordinary share	47,649,520	46,668,554	47,543,516	46,634,026

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 MONTHS ENDED JUNE 30, 2023 AND 2022

	Ordinary shares		Additional paid-in capital	Accumulated other comprehensive income / (loss)	Accumulated deficit	Total shareholders' equity
	No. of shares	Amount				
	(in thousands, except share and per share amounts)					
Balance at March 31, 2022	**46,641,448**	**$ 2,821**	**$ 1,084,306**	**$ (39,306)**	**$ (501,820)**	**$ 546,001**
Loss for the period	—	—	—	—	(39,061)	(39,061)
Other comprehensive loss	—	—	—	(28,324)	—	(28,324)
Exercises of share options	4,470	—	29	—	—	29
Restricted share units distributed during the period	36,333	2	(2)	—	—	—
Share-based compensation expense	—	—	7,813	—	—	7,813
Issuance of ordinary shares relating to employee stock purchase plan	2,332	—	30	—	—	30
Balance at June 30, 2022	**46,684,583**	**$ 2,823**	**$ 1,092,176**	**$ (67,630)**	**$ (540,881)**	**$ 486,488**
Balance at March 31, 2023	**47,546,673**	**$ 2,869**	**$ 1,121,554**	**$ (52,494)**	**$ (659,158)**	**$ 412,771**
Loss for the period	—	—	—	—	(68,474)	(68,474)
Other comprehensive gain	—	—	—	54	—	54
Exercises of share options	2,427	—	34	—	—	34
Restricted and performance share units distributed during the period	150,720	8	(8)	—	—	—
Share-based compensation expense	—	—	8,894	—	—	8,894
Issuance of ordinary shares relating to employee stock purchase plan	2,511	0	41	—	—	41
Balance at June 30, 2023	**47,702,331**	**$ 2,877**	**$ 1,130,515**	**$ (52,440)**	**$ (727,632)**	**$ 353,320**

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 MONTHS ENDED JUNE 30, 2023 AND 2022

	Ordinary shares		Additional paid-in capital	Accumulated other comprehensive income / (loss)	Accumulated deficit	Total shareholders' equity
	No. of shares	Amount				
	(in thousands, except share and per share amounts)					
Balance at December 31, 2021	**46,298,635**	**$ 2,802**	**$ 1,076,972**	**$ (28,856)**	**$ (455,142)**	**$ 595,776**
Loss for the period	—	—	—	—	(85,739)	(85,739)
Other comprehensive loss	—	—	—	(38,774)	—	(38,774)
Exercises of share options	72,594	4	449	—	—	453
Restricted and performance share units distributed during the period	307,464	17	(17)	—	—	—
Share-based compensation expense	—	—	14,682	—	—	14,682
Issuance of ordinary shares relating to employee stock purchase plan	5,890	—	90	—	—	90
Balance at June 30, 2022	**46,684,583**	**$ 2,823**	**$ 1,092,176**	**$ (67,630)**	**$ (540,881)**	**$ 486,488**
Balance at December 31, 2022	**46,968,032**	**$ 2,838**	**$ 1,113,393**	**$ (58,291)**	**$ (581,931)**	**$ 476,009**
Loss for the period	—	—	—	—	(145,701)	(145,701)
Other comprehensive gain	—	—	—	5,851	—	5,851
Exercises of share options	12,482	1	120	—	—	121
Restricted and performance share units distributed during the period	716,811	38	(38)	—	—	—
Share-based compensation expense	—	—	16,955	—	—	16,955
Issuance of ordinary shares relating to employee stock purchase plan	5,006	0	85	—	—	85
Balance at June 30, 2023	**47,702,331**	**$ 2,877**	**$ 1,130,515**	**$ (52,440)**	**$ (727,632)**	**$ 353,320**

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,	
	2023	2022
	(in thousands)	
Cash flows from operating activities		
Net loss	$ (145,701)	$ (85,739)
Adjustments to reconcile net loss to net cash used in operating activities:		
Depreciation and amortization expense	5,115	4,103
Share-based compensation expense	16,955	14,682
Royalty financing agreement interest expense	3,154	-
Deferred tax income	(1,044)	(934)
Changes in fair value of contingent consideration and derivative financial instrument, net	1,212	1,371
Unrealized foreign exchange losses / (gains), net	944	(24,491)
Other items, net	(831)	(257)
Changes in operating assets and liabilities:		
Accounts receivable and contract asset, prepaid expenses, and other current assets and receivables	813	46,492
Inventories	(3,288)	(2,949)
Accounts payable	(1,212)	7,632
Accrued expenses, other liabilities, and operating leases	(11,416)	(1,299)
Net cash used in operating activities	(135,299)	(41,389)
Cash flows from investing activities		
Purchases of property, plant, and equipment	(3,416)	(8,637)
Proceeds on maturity of investment securities	52,234	-
Acquisition of uniQure France SAS, net of cash acquired	-	(822)
Net cash generated from / (used in) investing activities	48,818	(9,459)
Cash flows from financing activities		
Proceeds from royalty financing agreement	374,350	-
Payment of debt issuance costs	(4,288)	-
Proceeds from issuance of ordinary shares related to employee stock option and purchase plans	206	543
Net cash generated from financing activities	370,268	543
Currency effect on cash, cash equivalents and restricted cash	1,812	(5,382)
Net decrease in cash, cash equivalents and restricted cash	285,599	(55,687)
Cash, cash equivalents and restricted cash at beginning of period	231,173	559,353
Cash, cash equivalents and restricted cash at the end of period	**$ 516,772**	**$ 503,666**
Cash and cash equivalents	$ 513,598	$ 500,524
Restricted cash related to leasehold and other deposits	3,174	3,142
Total cash, cash equivalents and restricted cash	**$ 516,772**	**$ 503,666**
Supplemental cash flow disclosures:		
Cash paid for interest	$ (8,882)	$ (3,980)
Non-cash (decrease) / increase in accounts payables and accrued expenses and other current liabilities related to purchases of property, plant, and equipment	$ (651)	$ (277)

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

1 General business information

uniQure N.V. (the "Company") was incorporated on January 9, 2012, initially 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 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 on the Nasdaq Global Select Market, the Company converted into a public company with limited liability (*naamloze vennootschap*) and changed its legal name from uniQure B.V. to uniQure N.V.

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

The Company's 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 United States Securities and Exchange Commission (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 United States ("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 three and six months ended June 30, 2023, are not necessarily indicative of the results to be expected for the full year ending December 31, 2023, 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, 2022 filed by the Company with the SEC on February 27, 2023 (the "Annual Report").

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, 2022, and the notes thereto, which are included in the Annual Report. There have been no material changes in the Company's significant accounting policies during the six months ended June 30, 2023, except as noted below.

Royalty Financing Agreement

In May 2023, uniQure biopharma B.V. ("uniQure biopharma"), a wholly-owned subsidiary of the Company entered into an agreement (the "Royalty Financing Agreement") with HemB SPV (the "Purchaser") to sell certain current and future royalties due to uniQure biopharma from CSL Behring LLC ("CSL Behring") under the Commercialization and License Agreement ("the CSL Behring Agreement") by and between uniQure biopharma and CSL Behring from the net sales of HEMGENIX®. Refer to Note 9 *"Royalty Financing Agreement"* for further details of the Royalty Financing Agreement. The Company determined that the Royalty Financing Agreement should be accounted for as debt in accordance with topic *ASC 470, Debt*. The Company initially recognized the debt at fair value. The Company subsequently records the debt at amortized cost and determines the effective interest rate based on its projection of contractual cash flows. Interest expense (presented as "Interest Expense" in the consolidated statements of operations and comprehensive loss) is recorded over the projected repayment period using the effective interest method. The Company periodically assesses and adjusts the effective interest rate to reflect changes in projected cash flows. The Company prospectively applies the adjusted effective interest rate following the date of change.

In accordance with topic *ASC 835, Interest*, debt issuance costs incurred in relation to the Royalty Financing Agreement are presented as a reduction of carrying amount of the debt. Debt issuance cost is amortized together with the interest expense recorded.

2.5 Recent accounting pronouncements

There have been no new accounting pronouncements or changes to accounting pronouncements during the six months ended June 30, 2023, as compared to the recent accounting pronouncements described in Note 2.3.25 of the Annual Report, which could be expected to materially impact the Company's unaudited consolidated financial statements.

3 CSL Behring collaboration

On June 24, 2020, uniQure biopharma B.V. entered into the CSL Behring Agreement with CSL Behring, pursuant to which CSL Behring received exclusive global rights to HEMGENIX®.

The transaction became fully effective on May 6, 2021.

License revenue

The Company recognized $0.8 million of royalty revenue in each of the three and six months ended June 30, 2023, compared to nil in the three and six months ended June 30, 2022. Royalties on the sale of the HEMGENIX® are recorded once earned and are presented as license revenue.

Accounts receivable and contract asset

As of December 31, 2022, the Company recorded accounts receivable of $2.2 million from CSL Behring related to collaboration services as well as a contract asset of $100.0 million for a milestone due from CSL Behring following the first sale of HEMGENIX® in the U.S., which was deemed to be probable.

As of June 30, 2023, the Company had accounts receivable of $102.5 million from CSL Behring. The $100.0 million milestone is included in accounts receivable as of June 30, 2023 following the achievement of the milestone event in June 2023. The Company collected the $100.0 million in July 2023. The remaining accounts receivable related to collaboration services, contract manufacturing revenue, and royalty revenue.

4 Investment securities

The following tables summarize the Company's investments in sovereign debt as of June 30, 2023 and December 31, 2022:

	At June 30, 2023			
	Amortized cost, as adjusted	Gross unrealized holding gains	Gross unrealized holding losses	Estimated fair value
	(in thousands)			
Current investments:				
Government debt securities (held-to-maturity)	$ 114,989	$ —	$ (363)	$ 114,626
Total	**$ 114,989**	**$ —**	**$ (363)**	**$ 114,626**

	At December 31, 2022			
	Amortized cost, as adjusted	Gross unrealized holding gains	Gross unrealized holding losses	Estimated fair value
	(in thousands)			
Current investments:				
Government debt securities (held-to-maturity)	$ 124,831	$ —	$ (283)	$ 124,548
Non-current investments:				
Government debt securities (held-to-maturity)	39,984	—	(43)	39,941
Total	**$ 164,815**	**$ —**	**$ (326)**	**$ 164,489**

Inputs to the fair value of the investments are considered Level 2 inputs.

5 Inventories

The following table summarizes the inventory balances as of June 30, 2023 and December 31, 2022:

	June 30, 2023	December 31, 2022
	(in thousands)	
Raw materials	$ 6,013	$ 3,584
Work in progress	4,059	1,874
Finished goods	140	1,466
Inventories	**$ 10,212**	**$ 6,924**

6 Fair value measurement

The Company measures certain financial assets and liabilities at fair value, either upon initial recognition or for subsequent accounting or reporting. *ASC 820, Fair Value Measurements and Disclosures* 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, accounts receivable from licensing and collaboration partners, 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, 2023, and December 31, 2022:

	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, 2022					
Assets:					
Cash and cash equivalents	$ 228,012	$ —	$ —	$ 228,012	Cash and cash equivalents
Restricted cash	3,161	—	—	3,161	Other non-current assets
Total assets	$ 231,173	$ —	$ —	$ 231,173	
Liabilities:					
Contingent consideration	—	—	35,316	35,316	Contingent consideration
Consideration for post-acquisition services	—	—	297	297	Other non-current liabilities
Total liabilities	**$ —**	**$ —**	**$ 35,613**	**$ 35,613**	
At June 30, 2023					
Assets:					
Cash and cash equivalents	$ 513,598	$ —	$ —	$ 513,598	Cash and cash equivalents
Restricted cash	3,174	—	—	3,174	Other non-current assets
Total assets	$ 516,772	$ —	$ —	$ 516,772	
Liabilities:					
Contingent consideration	—	—	37,247	37,247	Contingent consideration
Consideration for post-acquisition services	—	—	326	326	Other non-current liabilities
Total liabilities	**$ —**	**$ —**	**$ 37,573**	**$ 37,573**	

Contingent consideration

The Company is required to pay up to EUR 178.8 million (or $194.6 million based on the foreign exchange rate on June 30, 2023) to the former shareholders of Corlieve Therapeutics SAS ("uniQure France SAS") upon the achievement of contractually defined milestones in connection with the Company's July 2021 acquisition of uniQure France SAS.

The fair value of the contingent consideration as of June 30, 2023 was $37.2 million (December 31, 2022: $35.3 million) using discount rates of approximately 15.0% to 15.3% (December 31, 2022: 14.0% to 14.4%) as well as a 66.0% (December 31, 2022: 66.0%) likelihood of the target candidate for treatment of temporal lobe epilepsy ("AMT-260") advancing into clinical development by no later than late 2023. If as of June 30, 2023 the Company had assumed a 100% likelihood of AMT-260 advancing into clinical development, then the fair value of the contingent consideration would have increased to $51.5 million. If as of June 30, 2023 the Company had assumed that it would discontinue development of the AMT-260 program, then the contingent consideration would be released to income. Changes in fair value of the contingent consideration are recognized within research and development expenses in the consolidated statements of operations and comprehensive loss.

The following table presents the changes in fair value of contingent consideration between December 31, 2022 and June 30, 2023:

	Amount of contingent consideration 2023 (in thousands)
Balance at December 31, 2022	**$ 35,316**
Change in fair value (presented within research and development expenses)	1,212
Currency translation effects	719
Balance at June 30, 2023	**$ 37,247**

As of June 30, 2023, the Company classified $27.7 million of the total contingent consideration of $37.2 million as current liabilities. The balance sheet classification between current and non-current liabilities is based upon the Company's best estimate of the timing of settlement of the remaining relevant milestones.

Investment securities

Refer to Note 4 "*Investment securities*" for the fair value of the investment securities as of June 30, 2023 and December 31, 2022.

7 Accrued expenses and other current liabilities

Accrued expenses and other current liabilities include the following items:

	June 30, 2023	December 31, 2022
	(in thousands)	
Accruals for goods received from and services provided by vendors-not yet billed	$ 10,454	$ 11,120
Personnel related accruals and liabilities	12,312	17,201
Liability owed to the Purchaser pursuant to the Royalty Financing Agreement	771	—
Accrued contract fulfillment costs and costs to obtain a contract	—	2,250
Total	**$ 23,537**	**$ 30,571**

8 Long-term debt

On June 14, 2013, the Company entered into a venture debt loan facility with Hercules Capital, Inc. (formerly known as Hercules Technology Growth Capital, Inc.) ("Hercules"). The facility was amended and restated in 2014, 2016, 2018, January 2021, December 2021 (the "2021 Restated Facility") and on May 12, 2023 (the "2023 Amended Facility").

Upon entering into the 2021 Restated Facility, the Company drew down an additional $30.0 million, resulting in total principal outstanding of $100.0 million.

The 2023 Amended Facility extends the maturity date and interest-only period from December 1, 2025 to January 5, 2027 (the "Maturity Date").

The Company is required to repay the entire principal balance on the Maturity Date. The interest rate is adjustable and is the greater of (i) 7.95% and (ii) 7.95% plus the prime rate less 3.25% per annum. The Company paid a $2.5 million back-end fee in June 2023. Under the 2023 Amended Facility, the Company owes a back-end fee of $4.9 million on December 1, 2025 and a back-end fee of $1.3 million on the Maturity Date.

The amortized cost (including interest due presented as part of accrued expenses and other current liabilities) of the 2023 Amended Facility was $102.2 million as of June 30, 2023, compared to $103.8 million as of December 31, 2022, and is recorded net of discount and debt issuance costs. The foreign currency gain on the facility in the three and six months ended June 30, 2023 was $0.8 million and $1.6 million, respectively, compared to a foreign currency loss of $6.3 million and $8.4 million during the same period in 2022.

Interest expense associated with the 2023 Amended Facility during the three and six months ended June 30, 2023 was $3.7 million and $7.3 million, respectively, compared to $2.6 million and $5.0 million during the same period in 2022.

Under the 2023 Amended Facility the Company must remain current in its periodic reporting requirements and is required to keep a minimum cash balance deposited in bank accounts in the U.S. 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. Beginning on April 1, 2024, the Company is required to keep a minimum of unrestricted cash equal to at least 30% of the loan amount outstanding. In combination with other covenants, the 2023 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 to its shareholders. The Company secured the facilities by directly or indirectly pledging its total assets of $942.8 million, less $3.9 million of cash and cash equivalents and other current assets held by the Company, $86.7 million of other current assets and investment held by uniQure France SAS as well as receivables sold to the Purchaser.

Under the 2023 Amended Facility, the occurrence of a material adverse effect, as defined therein, would entitle Hercules to declare all principal, interest and other amounts owed by the Company immediately due and payable. As of June 30, 2023, the Company was in material compliance with all covenants and provisions.

9 Royalty Financing Agreement

On May 12, 2023, the Company entered into the Royalty Financing Agreement with the Purchaser. Under the terms of the Royalty Financing Agreement the Company received an upfront payment of $375.0 million in exchange for its rights to the lowest royalty tier on CSL Behring's worldwide net sales of HEMGENIX® for certain current and future royalties due to us. The Company is also eligible to receive an additional $25.0 million milestone payment under the Royalty Financing Agreement if 2024 net sales of HEMGENIX® exceed certain thresholds, as set forth in the Royalty Financing Agreement. The Purchaser will receive 1.85 times the upfront payment (or $693.8 million) and 1.85 times the $25.0 million milestone payment (if paid) until June 30, 2032 ("First Hard Cap Date") if such thresholds are met or, if such cap is not met by June 30, 2032, up to 2.25 times of the upfront and milestone payment (if paid) through December 31, 2038. If 2024 net sales do not exceed the pre-specified threshold, the Company will be obligated to pay $25.0 million to the Purchaser but only to the extent that the Company achieves a future sales milestone under the CSL Behring Agreement. If such milestone payment is not due from CSL Behring, the Company is not obligated to pay any amounts to the Purchaser.

The Company has retained the rights to all other royalties, as well as contractual milestones totaling up to $1.5 billion, under the terms of the CSL Behring Agreement.

Net proceeds from the Royalty Financing Agreement, after deducting professional and financial advisory fees related to the transaction of $4.9 million, were $370.1 million. The Company initially recorded these net proceeds as "Liability from royalty financing agreement" on its balance sheet as of closing of the transaction on June 5, 2023. Following the initial recognition, the Company records the debt at amortized cost.

The Company expects to satisfy its commitment to Purchaser prior to the First Hard Cap Date. The Company will record the difference of $323.7 million between the total expected payments of $693.8 million to the Purchaser and the $370.1 million net proceeds as interest expense using the effective interest rate method. The Company determined the effective interest rate based on the projected cash flows up to the First Hard Cap Date. Based on the Company's projections the effective interest rate is expected to be within a range of 12.0% per annum to 13.5% per annum. The Company would have recorded $3.1 million and $3.5 million of interest expense, with a rate of 12.0% and 13.5% respectively, for the three and six months ended June 30, 2023. The Company will prospectively update the effective interest rate at each reporting date based on updated projections.

The liability was initially recognized at fair value and inputs were considered Level 3 inputs.

The following table presents the movement in the liability related to the Royalty Financing Agreement between the closing of the transaction on June 5, 2023 and June 30, 2023:

	Amount of liability
	(in thousands)
Gross proceeds from royalty financing agreement on June 5, 2023	$ 375,000
Debt issuance costs paid	(4,938)
Liability owed to the Purchaser (presented as "Accrued expense and other current liabilities")	(771)
Interest expense for the period June 5, 2023 to June 30, 2023	3,154
Liability related to the royalty financing agreement	**$ 372,445**

10 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"). The number of shares authorized for issuance under the 2014 Plan is 12,601,471. The 2014 Plan expires on January 9, 2024.

In June 2018, the Company's shareholders adopted and approved an employee share purchase plan (the "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.

2014 Plans and ESPP

Share-based compensation expense recognized by classification included in the Consolidated Statements of Operations and Comprehensive Loss in relation to the 2014 Plans and the ESPP for the periods indicated below was as follows:

	Three months ended June 30,		Six months ended June 30,	
	2023	2022	2023	2022
	(in thousands)		(in thousands)	
Cost of manufacturing services revenue	$ 138	$ 148	$ 162	$ 148
Research and development	4,732	4,306	9,037	8,360
Selling, general and administrative	4,024	3,359	7,756	6,174
Total	**$ 8,894**	**$ 7,813**	**$ 16,955**	**$ 14,682**

Share-based compensation expense recognized by award type for the 2014 Plans as well as the ESPP was as follows:

	Three months ended June 30,		Six months ended June 30,	
	2023	2022	2023	2022
	(in thousands)		(in thousands)	
Award type/ESPP				
Share options	$ 3,485	$ 3,438	$ 6,759	$ 6,693
Restricted share units	5,388	4,127	10,018	7,397
Performance share units	12	242	162	580
Employee share purchase plan	9	6	16	12
Total	**$ 8,894**	**$ 7,813**	**$ 16,955**	**$ 14,682**

As of June 30, 2023, the unrecognized share-based compensation expense related to unvested awards under the 2014 Plans were:

Award type	Unrecognized share-based compensation expense (in thousands)	Weighted average remaining period for recognition (in years)
Share options	$ 31,265	2.75
Restricted share units	41,298	2.21
Performance share units	—	—
Total	**$ 72,563**	**2.44**

The Company satisfies the exercise of share options and vesting of Restricted Share Units ("RSUs") and Performance Share Units ("PSUs") through newly issued ordinary shares.

Share options

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% of each grant vests after one year from the initial grant date and the remainder vests in equal quarterly installments over years two, three and four. Certain grants to non-executive directors vest in full after one year. All share options must be exercised by the tenth anniversary of the initial grant date.

The following tables summarize share option activity under the 2014 Plans for the six months ended June 30, 2023:

	Options	
	Number of ordinary shares	Weighted average exercise price
Outstanding at December 31, 2022	**4,237,917**	**$ 26.13**
Granted	1,484,930	$ 19.40
Forfeited	(236,145)	$ 22.51
Expired	(61,142)	$ 52.43
Exercised	(12,482)	$ 9.72
Outstanding at June 30, 2023	**5,413,078**	**$ 24.18**
Thereof, fully vested, and exercisable on June 30, 2023	2,748,537	$ 26.61
Thereof, outstanding and expected to vest after June 30, 2023	2,664,540	$ 21.67

Total weighted average grant date fair value of options issued during the period (in $ millions)	$ 16.8
Proceeds from option sales during the period (in $ millions)	$ 0.1

The fair value of each share 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	**2023**	**2022**	**2023**	**2022**
Expected volatility	70%	70%	70%	70%
Expected terms	10 years	10 years	10 years	10 years
Risk free interest rate	3.71% - 4.00%	3.03% - 3.44%	3.71% - 4.10%	2.12% - 3.44%
Expected dividend yield	0%	0%	0%	0%

RSUs

The following table summarizes the RSU activity for the six months ended June 30, 2023:

	RSUs	
	Number of ordinary shares	Weighted average grant-date fair value
Non-vested at December 31, 2022	**1,818,774**	**$ 20.46**
Granted	1,516,025	$ 19.68
Vested	(662,393)	$ 21.85
Forfeited	(183,734)	$ 19.52
Non-vested at June 30, 2023	**2,488,672**	**$ 19.68**
Total weighted average grant date fair value of RSUs granted during the period (in $ millions)		$ 29.8

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

PSUs

The following table summarizes the PSU activity for the six months ended June 30, 2023:

	PSUs	
	Number of ordinary shares	Weighted average grant-date fair value
Non-vested at December 31, 2022	**400,690**	**$ 28.82**
Vested	(54,550)	$ 26.45
Forfeited	(38,260)	$ 27.85
Non-vested at June 30, 2023	**307,880**	**$ 29.36**

The Company granted ordinary shares to certain employees in September and December 2021 and at various dates during the year ended December 31, 2022 that will be earned upon the achievement of defined milestones. Such ordinary shares will vest upon the later of a minimum service period of one year or three years, or the achievement of defined milestones, subject to the grantee's continued employment. In addition, portions of the ordinary shares granted in December 2021 to executives and other members of senior management are subject to achieving a minimum total shareholder return relative to the NASDAQ Biotechnology Index. The Company recognizes the compensation cost related to these grants to the extent it considers achievement of the milestones to be probable. Achievement of one of the total five defined milestones was met as of December 31, 2022 and another one of the total five defined milestones was met as of June 30, 2023. The remaining three milestones have not yet been met.

The ESPP

During the six months ended June 30, 2023, 5,006 ordinary shares were issued under the ESPP compared to 5,890 during the same period in 2022. As of June 30, 2023, 111,054 ordinary shares remain available for issuance under the ESPP compared to a total of 121,414 as of June 30, 2022.

11 Income taxes

The Company recorded $0.2 million deferred tax loss and $1.0 million deferred tax benefit in relation to its operations in the U.S. and France during the three and six months ended June 30, 2023, respectively. The Company recorded $0.3 million and $0.9 million deferred tax benefit in relation to its operations in the U.S. and France during the three and six months ended June 30, 2022, respectively.

The effective income tax rate of 0.2% and (0.7%) during the three and six months ended June 30, 2023 is substantially lower than the enacted rate of 25.8% in the Netherlands as the Company records a valuation allowance against its net deferred tax assets in the Netherlands. The effective income tax rate during the three and six months ended June 30, 2022 was (0.8%) and (1.1%), respectively, as the Company had recorded a valuation allowance against its net deferred tax assets in the Netherlands.

12 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 in the three and six months ended June 30, 2023, all potentially dilutive ordinary shares would have an antidilutive effect, if converted, and thus have been excluded from the computation of loss per share for the three and six months ended June 30, 2023. The ordinary 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, 2023 and June 30, 2022, respectively.

The potentially dilutive ordinary shares are summarized below:

	Three months ended June 30,		Six months ended June 30,	
	2023	2022	2023	2022
Anti-dilutive ordinary share equivalents				
Stock options under 2014 Plans and previous plan	5,297,277	4,420,474	5,297,277	4,420,474
Non-vested RSUs and PSUs	2,730,672	2,447,570	2,730,672	2,447,570
ESPP	2,455	1,464	2,455	1,464
Total anti-dilutive ordinary share equivalents	**8,030,404**	**6,869,508**	**8,030,404**	**6,869,508**

13 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. 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 deliver to patients suffering from rare and other devasting diseases single treatments with potentially curative results. We are advancing a focused pipeline of innovative gene therapies, including our clinical candidates for the treatment of Huntington's disease and amyotrophic lateral sclerosis ("ALS"), as well as preclinical product candidates including candidates for the treatment of refractory temporal lobe epilepsy ("rTLE") and Fabry disease. In November 2022 and February 2023, our internally developed HEMGENIX®, a gene therapy for the treatment of hemophilia B, was approved for commercialization by the United States Food and Drug Administration (the "FDA") and the European Medicines Agency ("EMA"), respectively. In June 2020, we agreed to license HEMGENIX® to CSL Behring LLC ("CSL Behring"), pursuant to a commercialization and license agreement (the "Commercialization and License Agreement"), which is now responsible for commercialization of HEMGENIX®. We are manufacturing HEMGENIX® for CSL Behring and are entitled to specific milestone payments and royalties on net sales under the Commercialization and License Agreement. In May 2023, one of our wholly-owned subsidiaries entered into a royalty purchase agreement (the "Royalty Purchase Agreement") with HemB SPV, L.P. (the "Purchaser") for the sale of a portion of the royalty rights due to us from CSL Behring under the Commercialization and License Agreement.

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 Adeno-associated virus ("AAV") based gene therapies in our own facilities with a proprietary, commercial-scale, current good manufacturing practices 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:

Huntington's disease program (AMT-130)

AMT-130 is our novel gene therapy candidate for the treatment of Huntington's disease.

We are currently conducting a randomized, controlled, and blinded Phase I/II clinical trial for AMT-130 in the U.S. The low-dose cohort of this trial includes 10 patients, of which six patients received treatment with AMT-130 and four patients received imitation surgery. The high-dose cohort includes 16 patients, of which 10 patients received treatment with AMT-130 and six patients received imitation surgery. Patients in the high-dose cohort that received imitation surgery had the option to cross over after 12 months if they met the inclusion criteria for the study. We are also conducting an open-label Phase Ib/II study in the EU, which includes six patients in the low-dose cohort and nine patients in the high-dose cohort. All 15 patients in the EU study will receive AMT-130.

On March 21, 2022, we announced that we completed the enrollment of all 26 patients in the first two cohorts of our Phase I/II clinical trial of AMT-130 in the U.S. In July 2022, we began crossing over patients in the high-dose cohort who received the imitation surgical procedure. Four of the six control patients in the high-dose cohort have been crossed over to treatment (three patients received the high dose and one patient received the low dose). The remaining two control patients in the high-dose cohort did not meet all the inclusion criteria for the study and were not eligible for crossover. All four crossover patients received a short course of immunosuppression therapy concurrent with the administration of AMT-130.

On June 23, 2022, we announced that all six patients in the low-dose cohort in our Phase Ib/II study in the EU had been treated with AMT-130. We have continued to make progress enrolling the second, high-dose cohort of nine patients.

On June 21, 2023, we announced interim data, including up to 24-month follow-up, from 26 patients enrolled in the ongoing U.S. Phase I/II clinical trial of AMT-130. Efficacy and biomarker data from the crossover patients are not included in the summary below.

Safety and tolerability

We believe AMT-130 was generally well-tolerated, with a manageable safety profile in patients treated with the lower dose of $6x10^{12}$ vector genomes and the higher dose of $6x10^{13}$ vector genomes. The most common adverse events in the treatment groups were related to the surgical procedure. No treatment emergent adverse events led to discontinuation of patient follow-up.

As previously reported, there were two serious adverse events ("SAEs") unrelated to AMT-130 (post-operative delirium and major depression) in the low-dose cohort, one SAE in the high-dose cohort (back pain), and one SAE (deep vein thrombosis) in the control group. In addition, there were two suspected unexpected serious adverse events (severe headache, central nervous system inflammation) in the high-dose cohort. All the events have resolved to our knowledge.

Exploratory efficacy data

We believe early clinical data demonstrate promising trends. Compared to baseline measurements, clinical function was generally preserved at 24 months for patients in the low-dose cohort and at 12 months for patients in the high-dose cohort.

Compared to natural history, patients in both dose cohorts demonstrated benefits in each of Total Motor Score ("TMS"), Total Functional Capacity ("TFC") and the composite Unified Huntington's Disease Rating Scale ("cUHDRS"). Compared to natural history:

- TMS of patients in the lower dose cohort showed a mean improvement of 1.8 points at 24 months and patients in the higher dose cohort demonstrated a mean improvement of 2.7 points at 12 months;
- TFC of patients in the lower dose cohort showed a mean 0.8 point improvement at 24 months and patients in the higher dose cohort demonstrated a mean 0.5 point improvement at 12 months; and
- the cUHDRS of patients in the lower dose cohort showed a mean 0.9 point improvement in cUHDRS at 24 months and patients in the higher dose cohort demonstrated a mean 1.0 point improvement at 12 months.

Patients in the control group appear to experience a worsening of TMS at 12 months compared to baseline and natural history. TFC and cUHDRS was preserved in control patients at 12 months.

Biomarkers

Patients experienced a transient increase in neurofilament lights chain in the cerebral spinal fluid ("CSF NfL") related to the procedure that peaked at approximately one month after administration. These transient increases were not dose-dependent, and all patients experienced subsequent declines in CSL NfL. Mean CSF NfL for the lower dose cohort was 12.9% below baseline compared to a predicted 22.9% increase in the natural history, with four of the five patients in the lower dose cohort having CSF NfL levels below baseline. CSF NfL levels in the higher dose cohort were more variable through 12 months, with a mean increase of 51.5% compared to baseline. Four of the eight patients in the higher dose cohort with at least 12 months of follow-up had NfL levels below baseline. Two patients in the higher dose cohort with 18 months of follow-up demonstrated a continued decline in CSF NfL to 27.4% above baseline. In the control group, mean CSF NfL was relatively stable and was 6.83% below baseline at 12 months.

Mutant huntingtin protein in the cerebral spinal fluid ("CSF mHTT") for patients in the lower dose cohort remained below baseline with a mean reduction of 8.1% at 24 months. CSF mHTT for patients in the higher dose cohort was more variable with a mean increase of 39.7% above baseline at 12 months compared to a 4.7% increase in the control group. Three of nine evaluable patients in the higher dose cohort had CSF mHTT reduction below baseline at their last measurement. The mean total brain volume for the control, lower dose and higher dose cohorts declined 0.74%, 1.02% and 1.23%, respectively at 12 months and were not significantly different from each other or from the natural history.

The mean total brain volume for the control, low-dose and high-dose cohorts declined 0.74%, 1.02% and 1.23%, respectively at 12 months and do not appear to be significantly different from each other or from the natural history.

CSL Behring commercialization and license agreement

In June 2023, the first sale of HEMGENIX® in the U.S. occurred, and in July 2023 we collected the $100.0 million owed to us under the CSL Behring Agreement.

Financing

Royalty Financing Agreement

On May 12, 2023, we entered into the Royalty Financing Agreement with the Purchaser. Under the terms of the Royalty Financing Agreement, we received an upfront payment of $375.0 million in exchange for the Purchaser's rights to the lowest royalty tier on CSL Behring's worldwide net sales of HEMGENIX® for certain current and future royalties due to us. We are also eligible to receive an additional $25.0 million milestone payment under the Royalty Financing Agreement if 2024 net sales of HEMGENIX® exceed a pre-specified threshold. The Purchaser will receive 1.85 times the upfront payment (or $693.8 million) and 1.85 times the $25.0 million milestone payment (if paid) until June 30, 2032 ("First Hard Cap Date") or, if such cap is not met by June 30, 2032, up to 2.25 times the upfront and milestone payment (if paid) through December 31, 2038. If 2024 net sales do not exceed a pre-specified threshold, we will be obligated to pay $25.0 million to the Purchaser but only to the extent that we achieve a future sales milestone under the CSL Behring Agreement. If such milestone payment is not due from CSL Behring, we are not obligated to pay any amounts to the Purchaser.

We retained the rights to all other royalties, as well as contractual milestones totaling up to $1.5 billion, under the terms of the CSL Behring Agreement.

As a result of this transaction and the first U.S. sale of HEMGENIX® in which we collected $100.0 million owed to us, we believe our cash and cash equivalents will fund our operations through the second quarter of 2026.

Hercules Amendment

On May 12, 2023 we and Hercules amended the 2021 Restated Facility ("2023 Amended Facility"). The 2023 Amended Facility extends the maturity date and interest-only period from December 1, 2025 to January 5, 2027.

Amyotrophic Lateral Sclerosis (AMT-162)

On January 31, 2023, we announced that we had entered into a global licensing agreement with Apic Bio, Inc. ("Apic Bio") for a one-time, intrathecally administered investigational gene therapy for ALS caused by mutations in superoxide dismutase 1 ("SOD1"), a rapidly progressing, rare motor neuron disease that leads to loss of everyday functions and is uniformly fatal. With this agreement, we have added to our pipeline of gene therapies to treat neurological disorders. The FDA has cleared the investigational new drug application for APB-102 and has granted Orphan Drug and Fast Track designation. Mutations in the SOD1 gene of ALS account for approximately one-fifth of all inherited forms of this fatal disease. APB-102 is comprised of a recombinant AAVrh10 vector that expresses a micro ribonucleic acids ("miRNA") designed to knock down the expression of SOD1 with the goal of slowing down or potentially reversing the progression of ALS in patients with SOD1 mutations.

We made an initial cash payment of $10.0 million to Apic Bio that was recognized as a research and development expense. We owe up to $43.0 million in milestone payments to Apic Bio if AMT-162 is approved for commercialization in the U.S. and Europe.

Financial Overview

Key components of our results of operations include the following:

	Three months ended June 30,		Six months ended June 30,	
	2023	2022	2023	2022
	(in thousands)		(in thousands)	
Total revenues	$ 2,422	$ 497	$ 7,747	$ 2,289
Cost of contract manufacturing revenues	(1,352)	(832)	(3,787)	(832)
Research and development expenses	(46,036)	(46,192)	(106,845)	(91,195)
Selling, general and administrative expenses	(21,181)	(12,491)	(39,029)	(23,478)
Net loss	(68,474)	(39,061)	(145,701)	(85,739)

As of June 30, 2023 and December 31, 2022, we had cash and cash equivalents and investment securities of $628.6 million and $392.8 million, respectively. We had a net loss of $68.5 million and $145.7 million in the three and six months ended June 30, 2023, respectively, compared to net loss of $39.1 million and $85.7 million for the same period in 2022. As of June 30, 2023 and December 31, 2022, we had accumulated deficits of $727.6 million and $581.9 million, respectively.

We anticipate that our expenses will increase for the foreseeable future and will include costs related to:

- advancing AMT-130 for our Huntington's disease gene therapy program into phase III clinical study;
- advancing our gene therapy programs for rTLE, SOD1-ALS and Fabry disease into Phase I/II clinical studies;
- making potential future milestone payments related to the acquisition of Corlieve Therapeutics SAS ("uniQure France SAS"), if any; and
- potentially acquiring or in-licensing rights to new therapeutic targets, product candidates and technologies.

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 Securities and Exchange Commission (the "SEC") we make assumptions, judgments and estimates that can have a significant impact on our net loss and affect the reported amounts of certain assets, liabilities, revenue and expenses, and related disclosures. 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. A summary of our critical accounting policies as well as a discussion of our critical accounting estimates are presented in our Annual Report. There were no material changes to our critical accounting policies during the six months ended June 30, 2023 or reasonably possible changes of our critical accounting estimates as of June 30, 2023 that could have had a material impact on our results of operations for the three and six months ended June 30, 2023.

Cost of contract manufacturing

We entered into a development and commercial supply agreement with CSL Behring in June 2020. Since April 1, 2022, we recognize the cost to manufacture HEMGENIX® under such agreement as cost of contract manufacturing.

Research and development expenses

We expense research and development ("R&D") expenses as incurred. R&D expenses include costs which relate to our primary activities of biopharmaceutical research and development. 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 research activities for enabling technology platforms, such as next-generation vectors, promoters and re-administration of gene therapies;
- costs associated with the rendering of collaboration services;
- payments related to identifiable intangible assets without an alternative future use;
- payments to our licensors for milestones that have been achieved related to our product candidates, including approval of the marketing authorization application ("MAA");
- facilities, depreciation, and other expenses, which include direct and allocated expenses for rent and maintenance of facilities, insurance, and other supplies; and
- changes in the fair value of liabilities recorded in relation to our acquisition of uniQure France SAS.

Our R&D 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 the product candidates that we may develop could mean a significant change in the expenses and timing associated with the development of such product candidate.

Selling, general and administrative expenses

Our general and administrative expenses consist principally of employee, office, consulting, legal and other professional and administrative expenses. We incurred 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 advisory fees related to obtaining the CSL Behring Agreement.

Other items, net

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

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

Results of Operations

Comparison of the three months ended June 30, 2023 and 2022

The following table presents a comparison of our results of operations for the three months ended June 30, 2023 and 2022.

	Three months ended June 30,		
	2023	2022	2023 vs 2022
		(in thousands)	
Total revenues	$ 2,422	$ 497	$ 1,925
Operating expenses:			
Cost of contract manufacturing	(1,352)	(832)	(520)
Research and development expenses	(46,036)	(46,192)	156
Selling, general and administrative expenses	(21,181)	(12,491)	(8,690)
Total operating expenses	**(68,569)**	**(59,515)**	**(9,054)**
Other income	1,302	3,186	(1,884)
Other expense	(229)	(229)	—
Loss from operations	**(65,074)**	**(56,061)**	**(9,013)**
Other non-operating items, net	(3,237)	16,682	(19,919)
Net loss before income tax (expense) / benefit	**$ (68,311)**	**$ (39,379)**	**$ (28,932)**
Income tax (expense) / benefit	(163)	318	(481)
Net loss	**$ (68,474)**	**$ (39,061)**	**$ (29,413)**

Revenue

Our revenue for the three months ended June 30, 2023 and 2022 was as follows:

	Three months ended June 30,		
	2023	2022	2023 vs 2022
		(in thousands)	
License revenues	$ 793	$ —	$ 793
Contract manufacturing revenues	1,310	—	1,310
Collaboration revenues	319	497	(178)
Total revenues	**$ 2,422**	**$ 497**	**$ 1,925**

License revenue

We recognize royalty revenues from CSL Behring, related to HEMGENIX® sales, when earned. For the three months ended June 30, 2023, we recognized $0.8 million of license revenues (nil for the same period in 2022).

Contract manufacturing revenues

We recognize contract manufacturing revenue related to contract manufacturing HEMGENIX® for CSL Behring. Contract manufacturing revenue is realized when earned upon sales of HEMGENIX® to CSL Behring. We recognized $1.3 million contract manufacturing revenues in the three months ended June 30, 2023, compared to nil for the same period in 2022. We did not recognize such revenues in the three months ended June 30, 2022, as we started contract manufacturing activities to supply CSL Behring with launch supplies of HEMGENIX® following their submission of a Biologics License Application ("BLA") and MAA in the spring of 2022.

Collaboration revenues

We provide services to CSL Behring in accordance with the CSL Behring Agreement.

We entered into collaboration, research, and license agreements with Bristol-Myers Squibb ("BMS") in 2015 which were terminated on February 21, 2023.

For the three months ended June 30, 2023, we recognized $0.3 million and nil of collaboration revenue for CSL Behring and BMS, respectively. For the three months ended June 30, 2022, we recognized nil and $0.5 million of collaboration revenue for CSL Behring and BMS, respectively.

Cost of contract manufacturing

We incurred $1.4 million of cost of contract manufacturing related to the manufacture of HEMGENIX® in the three months ended June 30, 2023, compared to $0.8 million cost of contract manufacturing in the three months ended June 30, 2022.

R&D expenses

R&D expenses for the three months ended June 30, 2023 were $46.0 million, compared to $46.2 million for the same period in 2022. Other research and development expenses are separately classified in the table below. These other expenses are not allocated as they are deployed across multiple projects under development.

	Three months ended June 30,		
	2023	2022	2023 vs 2022
	(in thousands)		
Huntington's disease (AMT-130)	4,243	6,174	(1,931)
Temporal lobe epilepsy (AMT-260)	2,486	6,266	(3,780)
Amyotrophic lateral sclerosis (AMT-162)	$ 587	$ —	$ 587
Fabry disease (AMT-191)	400	485	(85)
Etranacogene dezaparvovec (AMT-060/061)	(2,003)	313	(2,316)
Programs in preclinical development and platform related expenses	2,257	1,740	517
Total direct research and development expenses	**$ 7,970**	**$ 14,978**	**$ (7,008)**
Employee and contractor-related expenses	17,760	15,066	2,694
Facility expenses	7,676	5,369	2,307
Disposables	5,875	4,532	1,343
Share-based compensation expense	4,732	4,306	426
Other expenses	1,786	2,137	(351)
Fair value changes related to contingent consideration	237	(196)	433
Total other research and development expenses	**$ 38,066**	**$ 31,214**	**$ 6,852**
Total research and development expenses	**$ 46,036**	**$ 46,192**	**$ (156)**

Direct research and development expenses

Huntington’s disease (AMT-130)

In the three months ended June 30, 2023 and June 30, 2022, our external costs for the development of AMT-130 were primarily related to the execution of our Phase I/II clinical trials in the United States and in Europe.

Temporal lobe epilepsy (AMT-260)

In the three months ended June 30, 2023 and June 30, 2022, we incurred $2.5 million and $6.3 million respectively, for the preclinical development of AMT-260. Our expenses in the three months ended June 30, 2022 included additional costs related to a toxicology study.

Amyotrophic Lateral Sclerosis caused by mutations in SOD1 (AMT-162)

On January 31, 2023, we entered into a global licensing agreement with Apic Bio for AMT-162. In the three months ended June 30, 2023, we incurred $0.6 million of costs (nil in the prior period), related to our preclinical activities for AMT-162.

Fabry disease (AMT-190)

In the three months ended June 30, 2023 and June 30, 2022, we incurred $0.4 million and $0.5 million of costs, respectively, related to our preclinical research of AMT-190.

Etranacogene dezaparvovec (AMT-060/061)

We have incurred external costs for our hemophilia B program related to the execution of our Phase III clinical trial and 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. CSL Behring is responsible for the clinical and regulatory activities and commercialization of the Product. However, we managed the trials on behalf of CSL Behring until such responsibilities were transitioned to CSL Behring in December 2022. Direct research and development expenses related to clinical development and other regulatory activities and commercialization expenses incurred in the three months ended June 30, 2023 and June 30, 2022 are presented net of reimbursements due from CSL Behring and include settlement amounts from the transition.

Preclinical programs & platform development

In the three months ended June 30, 2023, and June 30, 2022, we incurred $2.3 million and $1.7 million of costs, respectively, primarily related to our preclinical activities associated with product candidates for various other research programs and technology innovation projects.

Other research & development expenses

- We incurred $17.8 million in personnel and contractor-related expenses in the three months ended June 30, 2023, compared to $15.1 million for the same period in 2022. The increase was primarily a result of an increase in personnel and contractor-related expenses to support our growth;
- We incurred $7.7 million in operating expenses and depreciation expenses related to our rented facilities in the three months ended June 30, 2023, compared to $5.4 million in the same period in 2022. The increase primarily related to additional sites in Lexington, Massachusetts which commenced in May and November 2022;
- We incurred $5.9 million in disposable costs in the three months ended June 30, 2023, compared to $4.5 million for the same period in 2022;
- We incurred $4.7 million in share-based compensation expenses in the three months ended June 30, 2023, compared to $4.3 million for the same period in 2022;
- We incurred $1.8 million of other expenses for the three months ended June 30, 2023, compared to $2.1 million for the same period in 2022; and
- We incurred $0.2 million of expenses in the three months ended June 30, 2023 related to an increase in the fair value of contingent consideration associated with the acquisition of uniQure France SAS, compared to $0.2 million decrease in fair value for the same period in 2022.

Selling, general and administrative expenses

Selling, general and administrative expenses for the three months ended June 30, 2023 were $21.2 million, compared to $12.5 million for the same period in 2022.

- We incurred $6.4 million in personnel and contractor-related expenses in the three months ended June 30, 2023, compared to $5.2 million in the same period in 2022. The increase was primarily as a result of an increase in personnel and contractor-related expenses to support our growth;
- We incurred $4.0 million in share-based compensation expenses in the three months ended June 30, 2023, compared to $3.4 million in the same period in 2022. The increase was primarily a result of an increase in awards granted, including those to newly recruited personnel;
- We incurred $2.5 million in professional fees in the three months ended June 30, 2023, compared to $1.5 million in the same period in 2022. We regularly incur accounting, audit and legal fees associated with operating as a public company;
- We incurred $3.8 million in financial advisory fees in relation to our licensing transaction with CSL Behring in the three months ended June 30, 2023 (nil in prior period); and

- We incurred $3.2 million in other operating expenses in the three months ended June 30, 2023, compared to $1.9 million in the same period in 2022. The increase was primarily a result of an increase in information technology expenses to support operational improvements within the Company.

Other items, net

We recognized $1.2 million in other income from payments received from European authorities to subsidize our research and development efforts in the Netherlands in the three months ended June 30, 2023, compared to $2.6 million for the same period in 2022.

Other items, net for the periods presented primarily related to income from the subleasing of our Amsterdam facility and expenses we incur in relation to the subleasing facility.

Other non-operating items, net

Our other non-operating items, net, for the three months ended June 30, 2023 and 2022 were as follows:

	Three months ended June 30,		
	2023	2022	2023 vs 2022
		(in thousands)	
Interest income	$ 3,229	$ 35	$ 3,194
Interest expense	(6,840)	(2,694)	(4,146)
Foreign currency gains, net	374	19,398	(19,024)
Other non-operating losses	—	(57)	57
Total other non-operating income, net	**$ (3,237)**	**$ 16,682**	**$ (19,919)**

We recognize interest income associated with our cash and cash equivalents and investment securities. We recognized $3.2 million in interest income in the three months ended June 30, 2023, compared to $0.0 million in the same period in the prior year. Our interest income increased by $3.2 primarily due to the interest income earned on investment securities as well as cash on hand during the three months ended June 30, 2023.

We recognized $6.8 million in interest expense for the three months ended June 30, 2023 and $2.7 million for the three months ended June 30, 2022. Our interest expense in 2023 increased due to an increase in market interest rates related to the Hercules debt as well as the recognition of $3.2 million of interest expense related to the Royalty Financing Agreement.

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 recognized a net foreign currency gain, related to our borrowings from Hercules, the Royalty Financing Agreement and our cash and cash equivalents and investment securities as well as loans between entities within the uniQure group, of $0.4 million during the three months ended June 30, 2023, compared to a net gain of $19.4 million during the same period in 2022.

We recognized fair value changes related to the derivative financial liability related to a contingent payment due to BMS upon the consummation of a change of control transaction in the period ended June 30, 2022 (nil in the period ended June 30, 2023). As of December 31, 2022, we derecognized the derivative financial liability.

Income tax benefit

We recognized $0.2 million of deferred tax expense in the three months ended June 30, 2023, and $0.3 million of deferred tax benefit for the same period in 2022.

Comparison of the six months ended June 30, 2023 and 2022

The following table presents a comparison of our results of operations for the six months ended June 30, 2023 and 2022:

	Six months ended June 30,		
	2023	2022	2023 vs 2022
	(in thousands)		
Total revenues	$ 7,747	$ 2,289	$ 5,458
Operating expenses:			
Cost of contract manufacturing revenues	(3,787)	(832)	(2,955)
Research and development expenses	(106,845)	(91,195)	(15,650)
Selling, general and administrative expenses	(39,029)	(23,478)	(15,551)
Total operating expenses	**(149,661)**	**(115,505)**	**(34,156)**
Other income	3,113	3,496	(383)
Other expense	(445)	(422)	(23)
Loss from operations	**(139,246)**	**(110,142)**	**(29,104)**
Non-operating items, net	(7,499)	23,469	(30,968)
Loss before income tax benefit	$ **(146,745)**	$ **(86,673)**	**(60,072)**
Income tax benefit	1,044	934	110
Net loss	**$ (145,701)**	**$ (85,739)**	**$ (59,962)**

Revenue

Our revenue for the six months ended June 30, 2023 and 2022 was as follows:

	Six months ended June 30,		
	2023	2022	2023 vs 2022
	(in thousands)		
License revenues	$ 793	$ —	$ 793
Contract manufacturing revenues	$ 6,247	$ —	$ 6,247
Collaboration revenues	707	2,289	(1,582)
Total revenues	**$ 7,747**	**$ 2,289**	**$ 5,458**

License revenue

We recognize royalty revenues from CSL Behring, related to HEMGENIX® sales, when earned. For the six months ended June 30, 2023, we recognized $0.8 million of license revenue (nil for the same period in 2022).

Contract manufacturing revenues

We recognize contract manufacturing revenue related to contract manufacturing HEMGENIX® for CSL Behring. Contract manufacturing revenue is realized when earned upon sales of HEMGENIX® to CSL Behring. We recognized $6.2 million contract manufacturing revenues in the six months ended June 30, 2023, compared to nil for the same period in 2022. We did not recognize such revenues in the six months ended June 30, 2022, as we started contract manufacturing activities to supply CSL Behring with launch supplies of HEMGENIX® following their submission of a BLA and MAA in the spring of 2022.

Collaboration revenues

We provide services to CSL Behring in accordance with the CSL Behring Agreement.

We entered into collaboration, research, and license agreements with Bristol-Myers Squibb (“BMS”) in 2015 which were terminated on February 21, 2023.

For the six months ended June 30, 2023, we recognized $0.7 million and nil of collaboration revenue for CSL Behring and BMS, respectively. For the six months ended June 30, 2022, we recognized $1.4 million and $0.8 million of collaboration revenue for CSL Behring and BMS, respectively.

Cost of contract manufacturing

We incurred $3.8 million of cost of contract manufacturing related to the manufacture of HEMGENIX® in the six months ended June 30, 2023, compared to $0.8 million cost of contract manufacturing in the six months ended June 30, 2022.

R&D expenses

R&D expenses for the six months ended June 30, 2023 were $106.8 million, compared to $91.2 million for the same period in 2022. Other research and development expenses are separately classified in the table below. These other expenses are not allocated as they are deployed across multiple projects under development.

	Six months ended June 30,		
	2023	2022	2023 vs 2022
		(in thousands)	
Amyotrophic lateral sclerosis (AMT-162)	$ 10,628	$ —	$ 10,628
Huntington's disease (AMT-130)	7,976	11,391	(3,415)
Temporal lobe epilepsy (AMT-260)	7,308	8,895	(1,587)
Fabry disease (AMT-191)	1,488	1,115	373
Etranacogene dezaparvovec (AMT-060/061)	(1,336)	731	(2,067)
Programs in preclinical development and platform related expenses	4,142	3,033	1,109
Total direct research and development expenses	**$ 30,206**	**$ 25,165**	**$ 5,041**
Employee and contractor-related expenses	36,463	31,233	5,230
Facility expenses	14,462	10,668	3,794
Disposables	9,781	9,065	716
Share-based compensation expense	9,037	8,360	677
Other expenses	5,684	4,697	987
Fair value changes related to contingent consideration	1,212	2,007	(795)
Total other research and development expenses	**$ 76,639**	**$ 66,030**	**$ 10,609**
Total research and development expenses	**$ 106,845**	**$ 91,195**	**$ 15,650**

Direct research and development expenses

Amyotrophic Lateral Sclerosis caused by mutations in SOD1 (AMT-162)

On January 31, 2023, we entered into a global licensing agreement with Apic Bio for AMT-162. We have incurred $10.6 million expenses recorded to research and development expense in relation to the acquisition of assets without an alternative future use during the six-month period ended June 30, 2023.

Huntington’s disease (AMT-130)

In the six months ended June 30, 2023 and June 30, 2022, our external costs for the development of AMT-130 were primarily related to the execution of our Phase I/II clinical trials in the U.S. and in Europe.

Temporal lobe epilepsy (AMT-260)

In the six months ended June 30, 2023 and June 30, 2022, we incurred $7.3 million and $8.9 million respectively, for the preclinical development of AMT-260.

Fabry disease (AMT-190)

In the six months ended June 30, 2023 and June 30, 2022, we incurred $1.5 million and $1.1 million of costs, respectively, related to our preclinical research of AMT-190.

Etranacogene dezaparvovec (AMT-060/061)

We have incurred external costs for our hemophilia B program related to the execution of our Phase III clinical trial and 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. CSL Behring is responsible for the clinical and regulatory activities and commercialization of the Product. However, we managed the trials on behalf of CSL Behring until such responsibilities were transitioned to CSL Behring in December 2022. Direct research and development expenses related to clinical development and other regulatory activities and commercialization expenses incurred in the six months ended June 30, 2023 and June 30, 2022 are presented net of reimbursements due from CSL Behring and include settlement amounts from the transition.

Preclinical programs & platform development

In the six months ended June 30, 2023, and June 30, 2022, we incurred $4.1 million and $3.0 million of costs, respectively, primarily related to our preclinical activities associated with product candidates for various other research programs and technology innovation projects.

Other research & development expenses

- We incurred $36.5 million in personnel and contractor-related expenses in the six months ended June 30, 2023, compared to $31.2 million for the same period in 2022. The increase was primarily a result of an increase in personnel and contractor-related expenses to support our growth;
- We incurred $14.5 million in operating expenses and depreciation expenses related to our rented facilities in the six months ended June 30, 2023, compared to $10.7 million in the same period in 2022. The increase primarily related to additional sites in Lexington which commenced in May and November 2022;
- We incurred $9.8 million in disposable costs in the six months ended June 30, 2023, compared to $9.1 million for the same period in 2022;
- We incurred $9.0 million in share-based compensation expenses in the six months ended June 30, 2023, compared to $8.4 million for the same period in 2022;
- We incurred $5.7 million of other expenses for the six months ended June 30, 2023, compared to $4.7 million for the same period in 2022. The increase primarily related to contractual payments of $3.1 million we owed to a licensor upon the Europeans Medicines Agency approval of HEMGENIX® in February 2023, which were partially offset by a reduction in consultant-related expenses; and
- We incurred $1.2 million of expenses in the six months ended June 30, 2023 related to an increase in the fair value of contingent consideration associated with the acquisition of uniQure France SAS, compared to $2.0 million for the same period in 2022.

Selling, general and administrative expenses

Selling, general and administrative expenses for the six months ended June 30, 2023 were $39.0 million, compared to $23.5 million for the same period in 2022.

- We incurred $12.4 million in personnel and contractor-related expenses in the six months ended June 30, 2023, compared to $9.5 million in the same period in 2022. The increase was primarily as a result of an increase in personnel and contractor-related expenses to support our growth;
- We incurred $7.8 million in share-based compensation expenses in the six months ended June 30, 2023, compared to $6.2 million in the same period in 2022. The increase was primarily a result of an increase in awards granted, including those to newly recruited personnel;
- We incurred $5.7 million in professional fees in the six months ended June 30, 2023, compared to $2.7 million in the same period in 2022. We regularly incur accounting, audit and legal fees associated with operating as a public company. The increase from the prior period includes an increase in professional fees related to our global licensing agreement with Apic Bio;
- We incurred $3.8 million in financial advisory fees in relation to our licensing transaction with CSL Behring in the six months ended June 30, 2023 (nil in prior period);
- We incurred $2.4 million in intellectual property fees including registration and professional fees in the six months ended June 30, 2023 compared to $0.7 million in the same period in 2022. The increase mainly related to an increase in professional fees; and

- We incurred $6.2 million in other operating expenses in the six months ended June 30, 2023, compared to $3.9 million in the same period in 2022. The increase was primarily a result of an increase in information technology expenses to support operational improvements within the Company.

Other items, net

We recognized $2.5 million in other income from payments received from European authorities to subsidize our research and development efforts in the Netherlands in the six months ended June 30, 2023, compared to $2.7 million for the same period in 2022.

Other items, net for the periods presented, primarily related to income from the subleasing of our Amsterdam facility and expenses we incur in relation to the subleasing facility.

Other non-operating items, net

Our other non-operating items, net, for the six months ended June 30, 2023 and 2022 were as follows:

	Six months ended June 30,		
	2023	2022	2023 vs 2022
	(in thousands)		
Interest income	$ 4,898	$ 78	$ 4,820
Interest expense	(10,402)	(5,210)	(5,192)
Foreign currency (losses) / gains, net	(1,995)	27,966	(29,961)
Other non-operating gains	—	635	(635)
Total non-operating (expense) / income, net	**$ (7,499)**	**$ 23,469**	**$ (30,968)**

We recognize interest income associated with our cash and cash equivalents and investment securities. We recognized $4.9 million in interest income in the six months ended June 30, 2023, compared to $0.1 million in the same period in the prior year. Our interest income increased by $4.8 million primarily due to the interest income earned on investment securities as well as cash on hand during the six months ended June 30, 2023.

We recognized $10.4 million in interest expense for the six months ended June 30, 2023 and $5.2 million for the six months ended June 30, 2022. Our interest expense in 2023 increased due to an increase in market interest rates related to the Hercules debt as well as the recognition of $3.2 million of interest expense related to the Royalty Financing Agreement.

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 recognized a net foreign currency loss, related to our borrowings from Hercules, the Royalty Financing Agreement and our cash and cash equivalents and investment securities as well as loans between entities within the uniQure group, of $2.0 million during the six months ended June 30, 2023, compared to a net gain of $28.0 million during the same period in 2022.

We recognized fair value changes related to the derivative financial liability related to a contingent payment due to BMS upon the consummation of a change of control transaction in the six months ended June 30, 2022 (nil in the same period ended June 30, 2023). As of December 31, 2022, we derecognized the derivative financial liability.

Income tax benefit

We recognized $1.0 million of deferred tax benefit in the six months ended June 30, 2023, and $0.9 million of deferred tax benefit for the same period in 2022.

Financial Position, Liquidity and Capital Resources

As of June 30, 2023, we had cash and cash equivalents, restricted cash, and investment securities of $631.8 million. 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, royalty monetization financings, distribution, and licensing arrangements. We believe that our cash and cash equivalents and investment securities will fund our operations into the second quarter of 2026. Our material cash requirements include the following contractual and other obligations:

Debt

As of June 30, 2023, we had an outstanding loan amount owed to Hercules Capital, Inc. ("Hercules") for an aggregate principal amount of $100.0 million. Future interest payments and financing fees associated with the loan total $53.4 million, with $13.2 million payable within 12 months. We are contractually required to repay the $100.0 million in full in January 2027.

Royalty Financing Agreement

In May 2023, we entered into the Royalty Financing Agreement with the Purchaser. Under the Royalty Financing Agreement, we received an upfront cash payment of $375.0 million in exchange for the lowest royalty tier on CSL Behring's worldwide net sales of HEMGENIX® for certain current and future royalties due to us, up to 1.85 times (or $693.8 million) of this upfront cash payment until June 30, 2032 or, if such cap is not met by June 30, 2032, up 2.25 times the upfront cash payment (or $843.8 million) through December 31, 2038.

We are eligible to receive an additional $25.0 million milestone payment if 2024 net sales of HEMGENIX® exceed a pre-specified level.

If 2024 net sales do not exceed the pre-specified threshold, we will be obligated to pay $25.0 million to the Purchaser but only to the extent that we achieve future sales milestones under the CSL Behring Agreement.

Leases

We entered into lease arrangements for facilities, including corporate, manufacturing and office space. As of June 30, 2023, we had fixed lease payment obligations of $56.7 million, with $8.2 million payable within 12 months.

Commitments related to uniQure France SAS acquisition (nominal amounts)

In relation to the uniQure France SAS acquisition, we entered into commitments to make payments to the former shareholders upon the achievement of certain contractual milestones. The commitments include payments related to post-acquisition services that we agreed to as part of the transaction. As of June 30, 2023, our commitment amounts include up to $43.5 million in potential milestone payments through Phase I/II development and $174.2 million in potential milestone payments associated with Phase III development and the approvals of AMT-260 in the U.S. and European Union. The timing of achieving these milestones and consequently the timing of payments, as well as whether the milestone will be achieved at all, is generally uncertain. These payments are owed in Euro and have been translated at the foreign exchange rate as of June 30, 2023, of $1.09/€1.00. As of June 30, 2023, we expect these obligations will become payable between 2023 and 2031. If and when due, up to 25% of the milestone payments can be settled with our ordinary shares.

Commitments related to licensors and financial advisors

We 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 BLA, approval by the FDA or product launch) or because of collecting payments related to our sale of the exclusive global rights to the Product to CSL Behring. We also owe payments to a financial advisor related to certain payments we will collect under the CSL Behring Agreement.

The table below summarizes our consolidated cash flow data for the six months ended:

	Six months ended June 30,	
	2023	2022
	(in thousands)	
Cash, cash equivalents and restricted cash at the beginning of the period	$ 231,173	$ 559,353
Net cash used in operating activities	(135,299)	(41,389)
Net cash generated from / (used in) investing activities	48,818	(9,459)
Net cash generated from financing activities	370,268	543
Foreign exchange impact	1,812	(5,382)
Cash, cash equivalents and restricted cash at the end of period	**$ 516,772**	$ **503,666**

We had previously incurred losses and cumulative negative cash flows from operations since our business was founded by our predecessor entity AMT Therapeutics Holding N.V. ("AMT") in 1998, except for generating income in 2021 after receiving the upfront payment upon closing of the CSL Behring Agreement in 2021. We continue to incur losses in the current period. We recorded a net loss of $68.5 million and $145.7 million in the three and six months ended June 30, 2023, respectively, compared to a net loss of $39.1 million and $85.7 million during the same period in 2022. As of June 30, 2023, we had an accumulated deficit of $727.6 million.

Sources of liquidity

From our first institutional venture capital financing in 2006 through the current period, we funded our operations primarily through private and public placements of equity securities, debt securities, payments from our collaboration partners as well as from selling a portion of royalties due from our collaboration partner CSL Behring. In May 2021, we received a $462.4 million cash payment due from CSL Behring. We have collected $55.0 million related to CSL Behring's global regulatory submissions for etranacogene dezaparvovec in March and April 2022, and $100.0 million in July 2023 related to the first sale milestone of HEMGENIX® in the U.S., and are eligible to receive additional milestone payments, as well as royalties (to the extent not owed to settle the liability from royalty financing) on net sales of HEMGENIX®.

On March 1, 2021, we entered into a Sales Agreement with SVB Leerink LLC ("SVB Leerink") with respect to an at the market ("ATM") offering program, under which we may, from time to time in our sole discretion, offer and sell through SVB Leerink, acting as agent, our ordinary shares, up to an aggregate offering price of $200.0 million, for as long as our Board is authorized to issue such ordinary shares. We will pay SVB Leerink a commission equal to 3% of the gross proceeds of the sales price of all ordinary shares sold through it as a sales agent under the Sales Agreement. We did not issue ordinary shares under the Sales Agreement for the six months ended June 30, 2023 and June 30, 2022.

On May 12, 2023 we and Hercules amended the 2021 Restated Facility. The 2023 Amended Facility extends the maturity date and interest-only period from December 1, 2025 to January 5, 2027.

We are required to repay the entire principal balance of $100.0 million on the maturity date. The interest rate is adjustable and is the greater of (i) 7.95% and (ii) 7.95% plus the prime rate less 3.25% per annum. Under the 2023 Amended Facility, we owe a back-end fee of $4.9 million on December 1, 2025 and a back-end fee of $1.3 million on the maturity date.

We are subject to certain covenants under the 2023 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 2023 Amended Facility may limit our ability to obtain debt financing. The 2023 Amended Facility permits us to issue up to $500.0 million of convertible debt.

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 financing, 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 $135.3 million for the six months ended June 30, 2023 and consisted of net loss of $145.7 million adjusted for non-cash items, including depreciation and amortization expense of $5.1 million, share-based compensation expense of $17.0 million, changes in the fair value of contingent consideration of $1.2 million, unrealized foreign exchange losses of $0.9 million, $3.2 million of interest expense related to the royalty financing and a change in deferred taxes of $1.0 million. Net cash generated from operating activities also included unfavorable changes in operating assets and liabilities of $15.1 million. There was a net decrease in accounts receivable, prepaid expenses, and other current assets and receivables of $0.9 million. There was an increase in inventory balances of $3.3 million. These changes also relate to a net decrease in accounts payable, accrued expenses, other liabilities, and operating leases of $12.6 million, primarily related to a decrease of $4.9 million from personnel related accruals.

Net cash used in operating activities was $41.4 million for the six months ended June 30, 2022 and consisted of a net loss of $85.7 million adjusted for non-cash items, including depreciation and amortization expense of $4.1 million, share-based compensation expense of $14.7 million, changes in the fair value of contingent consideration and the derivative financial liability of $1.4 million, unrealized foreign exchange gains of $24.5 million and a change in deferred taxes of $0.9 million. Net cash generated from operating activities also included favorable changes in operating assets and liabilities of $49.4 million. There was a net decrease in accounts receivable and contract asset, prepaid expenses, and other current assets and receivables of $46.5 million, primarily related to the collection of $55.0 million of the contract asset related to CSL milestones of $55.0 million in March 2022 and April 2022. These changes also relate to a net increase in accounts payable, accrued expenses, other liabilities, and operating leases of $6.3 million.

Net cash generated from / (used in) investing activities

In the six months ended June 30, 2023, we generated $48.8 million in our investing activities compared to using $9.5 million for the same period in 2022.

	Six months ended June 30,	
	2023	2022
	(in thousands)	
Proceeds from maturity of investment securities	$ 52,234	$ —
Build out of Amsterdam site	(1,294)	(6,428)
Build out of Lexington site	(2,122)	(2,209)
Acquisition of uniQure France SAS, net of cash acquired	—	(822)
Total investments	**$ 48,818**	**$ (9,459)**

During the six months ended June 30, 2023, we received $52.2 million from the repayment of investment securities (nil for six months ended June 30, 2022).

The build out of the Amsterdam, Netherlands and Lexington, Massachusetts sites consumed $1.3 million and $2.1 million, respectively, during the six months ended June 30, 2023, compared to $6.4 million and $2.2 million for the same period in 2022.

Net cash generated from financing activities

In the six months ended June 30, 2023, we generated $370.3 million from financing activities compared to $0.5 million for the same period in 2022.

	Six months ended June 30,	
	2023	2022
	(in thousands)	
Cash flows from financing activities		
Proceeds from royalty financing agreement, net of debt issuance costs	$ 370,062	$ -
Proceeds from issuance of shares related to employee stock option and purchase plans	206	543
Net cash generated from financing activities	**$ 370,268**	**$ 543**

In June 2023, we received $370.1 million net proceeds from the Royalty Financing Agreement.

During the six months ended June 30, 2023, we received $0.2 million from the exercise of options to purchase ordinary shares in relation to our 2014 Plans compared to $0.5 million for the same period in 2022.

Funding requirements

Our future capital requirements will depend on many factors, including but not limited to:

- contractual milestone payments and royalties we might be owed in accordance with the CSL Behring Agreement;
- earnout payments we might owe the former shareholders of uniQure France SAS, which are subject to the achievement of specific development and regulatory milestones;
- the scope, timing, results, and costs of our current and planned clinical trials, including those for AMT-130 in Huntington's disease;
- the scope, obligations and restrictions on our business related to our existing equity, debt or royalty monetization financings and underlying agreements;
- the extent to which we acquire or in-license other businesses, products, product candidates or technologies;
- the amount and timing of revenue, if any, we receive from manufacturing products for CSL Behring;
- 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 costs associated with maintaining quality compliance and optimizing our manufacturing processes, including the operating costs associated with our Lexington, Massachusetts manufacturing facility; and
- the costs associated with increasing the scale and capacity of our manufacturing capabilities.

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 the 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, 2023, have not materially changed from our market risks and our exposure to market risk discussed in Part II, Item 7A of our Annual Report.

Item 4. Controls and Procedures

Evaluation of Disclosure Controls and Procedures

Our management, with the participation of our chief executive officer ("CEO") and chief financial officer ("CFO"), 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, 2023. Based on such evaluation, our CEO and CFO concluded that as of June 30, 2023, 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 period covered by this Quarterly Report on Form 10-Q, 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.

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, 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.

Summary Risk Factors

The following is a summary of the principal risks associated with an investment in our ordinary shares:

- We have encountered, and may continue to encounter, delays in, and impediments to the progress of our clinical trials or failure to demonstrate the safety and efficacy of our product candidates.
- The price of our ordinary shares has been and may in the future be volatile and fluctuate substantially.
- We may not be successful in our efforts to use our gene therapy technology platform to build a pipeline of additional product candidates, and we may not be successful in our efforts to create innovative programs, platform technologies or other technologies to be competitive with others.
- We may not be successful in our efforts to in-license or acquire product candidates that align with our research and development strategy, and any such transactions may not achieve the expected cash flows or could result in additional costs and challenges.
- Our manufacturing facility is subject to significant government regulations and approvals. If we fail to comply with these regulations or to maintain these approvals our business could be materially harmed.
- We are exposed to a number of external factors such as competition, insurance coverage of and pricing and reimbursement for our product candidates that may adversely affect our product revenue and that may cause our business to suffer. We also have experienced and could continue to experience increased competition for, and compensation expenses associated with employee recruiting and employee retention, which could adversely affect our business.
- We rely, and expect to continue to rely, on third parties to conduct, supervise, and monitor our preclinical studies and clinical trials, and those third parties may not perform satisfactorily, including failing to meet deadlines for the completion of such trials or failing to comply with regulatory requirements.
- 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.
- 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.
- Our reliance on third parties may require us to share our trade secrets and other proprietary technology, which could increase the possibility that a competitor will discover them or that our trade secrets and other proprietary technology will be misappropriated or disclosed.

- 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 condition, results of operations, and cash flows. The amount of capital we require will depend in part on the future payments we receive from CSL Behring related to HEMGENIX® commercial supply, contractual milestones, and royalties on net sales (to the extent not owed to settle the liability from royalty financing).
- Our relationships with employees, customers, and third parties are subject to applicable laws and regulations, the non-compliance of any of which could 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 may have an adverse effect on our business, financial condition, and results of operations.
- Our internal computer systems, or those of our collaborators or other contractors or consultants, may fail or suffer security breaches or other errors or disruptions, which could result in a material disruption of our product development programs, such as potential issues with data integrity or loss of data.
- 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.

Risks Related to the Development of Our Product Candidates

Our product candidates in development have not yet 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.

Our pipeline consists of product candidates in research or development that have not been approved for commercial sale. We have not generated any revenue from the sale of products or manufacturing of a product for a third party related to our product candidates in development and do not expect to generate any such revenue this year. Our product candidates, including AMT-130 and any of our other potential product candidates, will require extensive preclinical and/or clinical testing, manufacture development 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 have encountered and may encounter future 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 the 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.

For example, we experienced an immaterial but unexpected delay when our clinical trials of HEMGENIX® were placed on clinical hold by the FDA from December 2020 to April 2021, following a preliminary diagnosis of hepatocellular carcinoma in one patient. Similarly, we experienced an unexpected delay in the enrollment of our Phase Ib/II clinical trial for Huntington’s disease between July and October 2022 because of our voluntary postponement and comprehensive safety investigation into suspected unexpected serious adverse reactions in three patients.

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, as well as product candidate approval, include, but are not limited to:

- occurrence of serious adverse events associated with a product candidate that are viewed to outweigh its potential benefits;
- 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 or failure to obtain required IRB and IBC approval at each clinical trial site;
- requirements of regulatory authorities, IRBs, or IBCs to modify a study in such a way that it makes the study impracticable to conduct;
- regulatory authority requirements to perform additional or unanticipated clinical trials;
- changes in standards of care which may necessitate the modification of our clinical trials or the conduct of new trials;
- regulatory authority refusal to accept data from foreign clinical study sites;
- disagreements with regulatory authorities regarding our study design, including endpoints, our chosen indication, or our interpretation of data from preclinical studies and clinical trials or a finding that a product candidate's benefits do not outweigh its safety risks;
- recommendations from DSMBs to discontinue, pause, or modify the trial;
- imposition of a clinical hold by regulatory agencies after an inspection of our clinical trial operations or trial sites;
- suspension or termination of clinical research for various reasons, including noncompliance with regulatory requirements or a finding that the participants are being exposed to unacceptable health risks, undesirable side effects, or other unexpected characteristics (alone or in combination with other products) of the product candidate, or due to findings of undesirable effects caused by a chemically or mechanistically similar therapeutic or therapeutic candidate;
- 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;
- failure of patients to abide by clinical trial requirements;
- difficulty or delays in patient recruiting into clinical trials or in the addition of new investigators;
- 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;
- the number of patients required for clinical trials of our product candidates being larger than we anticipate;
- clinical trials producing negative or inconclusive results, or our studies failing to reach the necessary level of statistical significance, requiring that we conduct additional clinical trials or abandon product development programs;
- interruptions in manufacturing clinical supply of our product candidates or issues with manufacturing product candidates that meet the necessary quality requirements;
- unanticipated clinical trial costs or insufficient funding, including paying substantial application user fees;
- occurrence of serious adverse events or other undesirable side effects associated with a product candidate that are viewed to outweigh its potential benefits;
- disagreements with regulatory authorities regarding the interpretation of our clinical trial data and results, or the emergence of new information about or impacting our product candidates;
- determinations that there are issues with our manufacturing facility or process; or
- changes in regulatory requirements and guidance, as well as new, revised, postponed, or frozen regulatory requirements (such as the EU Clinical Trials Regulation), 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 in 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 U.S. 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 trials 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 a new therapeutic such as a gene therapy and the possibility that treatment with a gene therapy therapeutic could preclude future gene therapy treatments due to the formation of antibodies following and in response to the treatment.

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. It is also possible that any such manufacturing or formulation changes may have an adverse impact on the performance of the product candidate. 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 condition, 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.

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. Changes to product candidates may also impact their performance in subsequent studies.

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.

Interim, "top-line" or preliminary data from studies or trials announced or published from time to time may change as more data become available and are subject to audit and verification procedures that could result in material changes in the final data.

From time to time, we publicly disclose "top-line" or preliminary data from preclinical studies and clinical trials, which are based on a preliminary analysis of then-available data, and the results and related findings and conclusions are subject to change following a more comprehensive review of the data, the particular study, or trial. We also make assumptions, estimations, calculations, and conclusions as part of our analyses of data, and we may not have received or had the opportunity to evaluate all data. As a result, the "top-line" or preliminary data that we report may differ from future results of the same studies, or different conclusions or considerations may qualify such results once additional data have been received and fully evaluated. "Top-line" or preliminary data also remain subject to audit and verification procedures that may result in the final data being materially different from the preliminary data we previously published. As a result, "top-line" or preliminary data should be viewed with caution until the final data are available.

From time to time, we also disclose interim data from our preclinical studies and clinical trials. Interim data from clinical trials that we may complete are subject to the risk that one or more of the clinical outcomes may materially change as patient enrollment continues and more patient data become available. Adverse differences between interim data and final data could seriously harm our business.

Third parties, including regulatory agencies, may not accept or agree with our assumptions, estimates, calculations, conclusions, or analyses or may interpret or weigh the importance of data differently, which could impact the value of the particular program, the approvability or commercialization of the particular product candidate or product and our company in general. In addition, the information we choose to publicly disclose regarding a particular study or clinical trial is based on what is typically extensive information, and you or others may not agree with what we determine is the material or otherwise appropriate information to include in our disclosure. Any information we determine not to disclose may ultimately be deemed significant by you or others with respect to future decisions, conclusions, views, activities or otherwise regarding a particular product candidate or our business. If the "top-line" or preliminary data that we report differ from final results, or if others, including regulatory authorities, disagree with the conclusions reached, our ability to obtain approval for, and commercialize, product candidates may be harmed, which could seriously harm our business.

Fast track product, breakthrough therapy, priority review, or 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 fast-track designations, 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. An 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, RMAT, 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 fast-track products, RMAT 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 and the FDA approves a schedule for the submission of the remaining sections. For products that receive a priority review designation, the FDA's marketing application review goal is shortened to six months, as opposed to ten months under standard review.

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 a 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, the FDA may later decide that the products no longer meet the applicable conditions for qualification as either a fast-track product, RMAT, or a breakthrough therapy or, for priority review products, decide that the period for FDA review or approval will not be shortened. Moreover, in the U.S., FDA expects that sponsors with products under these programs will be prepared for a more rapid pace of development, including with respect to manufacturing or any combination medical devices, such as companion diagnostics. If we are unable to meet these expectations, we may not be able to fully avail ourselves of certain advantages of these programs.

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. Due to the significant resources required for the development of our product candidates, we must decide which product candidates to pursue and advance and the resources to allocate to each. Our decisions concerning the allocation of research, development, collaboration, management, and financial resources toward particular product candidates may not lead to the development of any viable commercial product and may divert resources away from better opportunities. 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.

A small number of patients have experienced serious adverse events during our clinical trials of either AMT-060 (our first-generation hemophilia B gene therapy), etranacogene dezaparvovec, and AMT-130. However, 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 delay, a hold or termination of our clinical trials, 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.

Certain of our product candidates may require medical devices for product administration and/or diagnostics, resulting in our product candidates being deemed combination products or otherwise being dependent upon additional regulatory approvals. This may result in the need to comply with additional regulatory requirements. If we are unable to meet these regulatory requirements, we may be delayed or not be able to obtain product approval.

Certain of our product candidates, such as AMT-130, require medical devices, such as a stereotactic, magnetic resonance imaging guided catheter, for product administration. Other of our product candidates may also require the use of a companion diagnostic device to confirm the presence of specific genetic or other biomarkers.

It is possible that our product candidates would be deemed to be combination products, potentially necessitating compliance with the FDA's investigational device regulations, separate marketing application submissions for the medical device component, a demonstration that our product candidates are safe and effective when used in combination with the medical devices, cross-labeling with the medical device, and compliance with certain of the FDA's device regulations. If we are not able to comply with the FDA's device regulations, if we are not able to effectively partner with the applicable medical device manufacturers, if we or any partners are not able to obtain any required FDA clearances or approvals of the applicable medical devices, or if we are not able to demonstrate that our product candidates are safe and efficacious when used with the applicable medical devices, we may be delayed in or may never obtain FDA approval for our product candidates, which would materially harm our business.

Moreover, certain of our delivery modalities, such as direct delivery of product candidates to the brain, may require significant physician ability and skill. If physicians are not able to effectively deliver our product candidates to the applicable site of action or if delivery modalities are too difficult, we may never be able to obtain approval for our product candidates, may be delayed in obtaining approval, or, following approval, physicians may not adopt our product candidates, any of which may materially harm our business.

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 could be materially harmed.

Our manufacturing facility in Lexington is subject to ongoing regulation and periodic inspection by the FDA, EU member state, and other regulatory bodies to ensure compliance with current cGMP requirements. 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, EU member state, 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 or recommending product recalls or seizing products; imposing operating restrictions; and seeking criminal prosecutions, among other outcomes. Poor control of production processes can also lead to the introduction of adventitious agents or other contaminants, or to inadvertent changes in the properties or stability of a product candidate that may not be detectable in final product testing and that could have an adverse effect on clinical studies, or patient safety or efficacy. Moreover, if our manufacturing facility is not able to meet regulatory requirements, we may need to implement costly and time-consuming remedial actions. Any of the foregoing could materially harm our business, financial condition, and results of operations.

Moreover, if we are not able to manufacture a sufficient amount of our product candidates for clinical studies or eventual commercialization, our development program and eventual commercial prospects will be harmed. If we cannot produce an adequate amount of our product candidates in compliance with the applicable regulatory requirements, we may need to contract with a third party to do so, in which case third party manufacturers may not be available or available on favorable terms. The addition of a new manufacturer may also require FDA, EMA, EU, and other regulatory authority approvals, which we may not be able to obtain.

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 our manufacturing processes, which may result in delays in regulatory approvals, inability to produce sufficient amounts of commercial product, 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, war or cases of force majeure and acts of God (including the effects of the Covid 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 U.S. 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 validate our manufacturing processes and methods or develop new processes and methods to meet our product supply needs and obligations.

The manufacture of our AAV gene therapies is complex and requires significant expertise. Even with the relevant experience and expertise, manufacturers of gene therapy products often encounter difficulties in production, particularly in scaling out and validating initial production, and ensuring that the product meets required specifications. These problems include difficulties with production costs and yields, quality control, including stability and potency of the product, quality assurance testing, operator error, shortages of qualified personnel, as well as compliance with strictly enforced federal, state, and foreign regulations. In the past, we have manufactured certain batches of product candidates, intended for nonclinical, clinical and process validation purposes that have not met all our pre-specified quality parameters. To meet our expected future production needs and our regulatory filing timelines for gene therapy product candidates we will need to complete the validation of our manufacturing processes and methods, and we may need to develop and validate new or larger scale manufacturing processes and methods. If we are unable to consistently manufacture our gene therapy product candidates or any approved products in accordance with our pre-specified quality parameters and applicable regulatory standards, it could adversely impact our ability to validate our manufacturing processes and methods, to meet our production needs, to file a BLA or other regulatory submissions, to develop our other proprietary programs, to conserve our cash, or to receive financial payments pursuant to our agreements with third parties.

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 U.S., 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 and our ability to generate revenue will be materially impaired.

The process of obtaining marketing approval for our product candidates in the U.S., 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 may also be delayed in completing their review of any marketing applications submitted by us or our partners. 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.

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 several gene therapy product candidates under development, in the U.S., the FDA has only approved a limited number of gene therapy products, to date. Accordingly, regulators, like the FDA, may have limited experience with the review and approval of marketing applications for gene therapy products.

Both the FDA and the 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 U.S. or EU, 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 U.S., there have been a number of recent changes relating to gene therapy development. By example, FDA issued a number of new guidance documents, and continues to issue guidance documents, on human gene therapy development, one of which was specific to human gene therapy for hemophilia, one that was specific to neurodegenerative diseases, and another of which was specific to rare diseases. 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 their approaches 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 U.S. and the European Union, may designate drugs for relatively small patient populations as orphan drugs. While certain of our product candidates have received orphan drug designation, there is no guarantee that we will be able to receive such designations in the future. The FDA may grant orphan designation to multiple sponsors for the same compound or active molecule and for the same indication. If another sponsor receives FDA approval for such product before we do, we would be prevented from launching our product in the U.S. for the orphan indication for a period of at least seven years unless we can demonstrate clinical superiority.

Moreover, while orphan drug designation neither shortens the development or regulatory review time, nor gives the product candidate advantages in the regulatory review or approval process, 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 the EMA from approving another marketing application for the same drug for the same indication for that period. The FDA and the EMA, however, may subsequently approve a similar drug or same drug, in the case of the U.S., for the same indication during the first product's market exclusivity period if the FDA or the EMA concludes that the later drug is clinically superior in that it is shown to be safer or more effective or makes a major contribution to patient care. Orphan exclusivity in the U.S. also does not prevent the FDA from approving another product that is considered to be the same as our product candidates for a different indication or a different product for the same orphan indication. If another product that is the same as ours is approved for a different indication, it is possible that third-party payors will reimburse for products off-label even if not indicated for the orphan condition. Moreover, in the U.S. the exact scope of orphan drug exclusivity is currently uncertain and evolving due to a recent court decision.

Orphan drug exclusivity may be lost if the FDA or the 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 condition.

Additionally, regulatory criteria with respect to orphan products are evolving, especially in gene therapy. By example, in the U.S., whether two gene therapies are considered to be the same for the purpose of determining clinical superiority was recently updated via a final guidance document specific to gene therapies, and depends on a number of factors, including the expressed transgene, the vector, and other product or product candidate features. Depending on the products, whether two products are ultimately considered to be the same may be determined by FDA on a case-by-case basis, making it difficult to make predictions regarding when the FDA might be able to make an approval of a product effective and whether periods of exclusivity will effectively block competitors seeking to market products that are the same or similar to ours for the same intended use. Accordingly, whether any of our product candidates will be deemed to be the same as another product or product candidate is uncertain.

As appropriate, we intend to seek 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 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 be granted any such periods of market exclusivity. By example, regulatory authorities may determine that our product candidates are not eligible for periods of regulatory exclusivity for various reasons, including a determination by the FDA that a BLA approval does not constitute a first licensure of the product. 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 could be materially harmed, as we will potentially be subject to greater market competition and may lose the benefits associated with programs. It is also possible that periods of exclusivity will not adequately protect our product candidates from competition. For instance, even if we receive twelve years of exclusivity from the FDA, other applicants will still be able to submit and receive approvals for versions of our product candidates through a full BLA.

If we do not obtain or maintain periods of market exclusivity, we may face competition sooner than otherwise anticipated. For instance, in the U.S., this could mean that a competing biosimilar product may be able to apply to the FDA and obtain approval either as a biosimilar to one of our products or even as an interchangeable product. This may require that we undertake costly and time-consuming patent litigation, to the extent available, or defend actions brought by the biosimilar applicant for declaratory judgment. If a biosimilar product does enter the market, it is possible that it could be substituted for one of our product candidates, especially if it is available at a lower price.

It is also possible that, at the time we obtain approval of our product candidates, regulatory laws and policies around exclusivities may have changed. For instance, there have been efforts to decrease the U.S. period of exclusivity to a shorter timeframe. Future proposed budgets, international trade agreements and other arrangements or proposals may affect periods of exclusivity.

If any of our product candidates receive regulatory approval, we and/or our partners will be subject to extensive regulatory requirements. Failure to fulfill and comply with the applicable regulatory requirements could result in regulatory enforcement actions that would be detrimental to our business.

Following any regulatory approval, the FDA and the EMA may impose certain post-approval requirements related to a product. Specifically, any approved products will be subject to continuing and comprehensive regulation concerning the product's design, testing, manufacture, safety, efficacy, recordkeeping, labeling, storage, approval, advertising, promotion, sale, and distribution. Regulatory authorities may also require post-marketing testing, known as Phase 4 testing, a risk evaluation and mitigation strategy, and surveillance to monitor the effects of an approved product or place conditions on an approval that could restrict the distribution or use of the product. Failure to comply with any of these requirements could result in regulatory, administrative, or other enforcement action, which would be detrimental to our business.

For instance, the FDA and other government agencies closely regulate the post-approval marketing and promotion of approved products, including off-label promotion, industry-sponsored scientific and educational activities, and the Internet and social media. Approved products may be marketed only for the approved indications and in accordance with the provisions of the approved labeling. Failure to comply with regulatory promotional standards could result in actions being brought against us by these agencies.

Moreover, if a company obtains FDA approval for a product via the accelerated approval pathway, the company would be required to conduct a post-marketing confirmatory trial to verify and describe the clinical benefit in support of full approval. FDA can require that this confirmatory trial be commenced prior to FDA granting a product accelerated approval. An unsuccessful post-marketing study or failure to complete such a study could result in the expedited withdrawal of the FDA's marketing approval for a product using a statutorily defined streamlined process.

Changes to some of the conditions established in an approved application, including changes in labeling, indications, manufacturing processes or facilities, may require a submission to and approval by the FDA or the EMA, as applicable, before the change can be implemented. A New Drug Application ("NDA")/BLA or MAA supplement for a new indication typically requires clinical data similar to that in the original application. The applicable regulatory authorities would review such supplement using similar procedures and actions as in reviewing NDAs/BLAs and MAAs.

Adverse event reporting and submission of periodic reports is required following marketing approval. Regulatory authorities may withdraw product approvals or request product recalls, as well as impose other enforcement actions, if a company fails to comply with regulatory standards, if it encounters problems following initial marketing, or if previously unrecognized problems are subsequently discovered.

In addition, the manufacture, testing, packaging, labeling, and distribution of products after approval will need to continue to conform to cGMPs. Drug and biological product manufacturers and certain of their subcontractors are subject to periodic unannounced inspections by the FDA or the EMA for compliance with cGMPs. Accordingly, manufacturers must continue to expend time, money, and effort in the areas of production and quality control to maintain compliance with cGMPs. In addition, prescription drug manufacturers in the U.S. must comply with applicable provisions of the Drug Supply Chain Security Act and provide and receive product tracing information, maintain appropriate licenses, ensure they only work with other properly licensed entities and have procedures in place to identify and properly handle suspect and illegitimate products.

Where we partner with third parties for the development, approval, and marketing of a product, such third parties will be subject to the same regulatory obligations as we will. However, as we will not control the actions of the applicable third parties, we will be reliant on them to meet their contractual and regulatory obligations. Accordingly, actions taken by any of our partners could materially and adversely impact our business.

Risks Related to Commercialization

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

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

- successful completion of preclinical studies and clinical trials, and other work required by regulators;
- receipt and maintenance of marketing approvals from applicable regulatory authorities;
- obtaining and maintaining patent and trade secret protection and non-patent, exclusivities for our product candidates;

- 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 profiles;
- the strength of our marketing and distribution;
- achieve optimal pricing based on durability of expression, safety, and efficacy;
- the ultimate content of the regulatory authority approved label, including the approved clinical indications, and any limitations or warnings;
- any distribution or use restrictions imposed by regulatory authorities;
- the interaction of our products with any other medicines that patients may be taking or the restriction on the use of our products with other medicines;
- the standard of care at the time of product approval;
- the relative convenience and ease of administration of our products;
- obtaining healthcare coverage and adequate reimbursement of our products;
- any price concessions, rebates, or discounts we may need to provide;
- complying with any applicable post-approval commitments and 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.

Even if our product candidates are approved, they may be subject to limitations that make commercialization difficult. There may be limitations on the indicated uses and populations for which the products may be marketed. They may also be subject to other conditions of approval, may contain significant safety warnings, including boxed warnings, contraindications, and precautions, may not be approved with label statements necessary or desirable for successful commercialization, or may contain requirements for costly post-market testing and surveillance, or other requirements, including the submission of a risk evaluation and mitigation strategy ("REMS") to monitor the safety or efficacy of the products. Failure to achieve or implement any of the above 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 could 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.

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 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 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 U.S., 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, any of which could 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. Public and medical community adoption of any of our gene therapies will also depend on factors including the ease of administration in comparison to other therapeutics. By example, the need for complex surgeries for the administration of a product candidate may impact the acceptance of a product.

In particular, our success will depend upon physicians who specialize in the treatment of genetic diseases targeted by our product and product candidates, prescribing treatments that involve the use of our product and product candidates, 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 based on 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, or our commercial partners, obtain approval to commercialize any of our product candidates outside of the U.S., 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 U.S., 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 which may make it more difficult or expensive to export or import products and supplies to or from the U.S.;
- 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 U.S.;
- 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 may be adversely affected by the effects of inflation.

Inflation has the potential to adversely affect our liquidity, business, financial condition, and results of operations by increasing our overall cost structure. The existence of inflation in the economy has resulted in, and may continue to result in, higher interest rates and capital costs, shipping costs, supply shortages, increased costs of labor, weakening exchange rates and other similar effects. As a result of inflation, we have experienced, and may continue to experience, cost increases. Although we may take measures to mitigate the impact of this inflation, if these measures are not effective our business, financial condition, results of operations and liquidity could be materially adversely affected. Even if such measures are effective, there could be a difference between the timing of when these beneficial actions impact our results of operations and when cost inflation is incurred.

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 face worldwide competition from larger pharmaceutical companies, specialty pharmaceutical companies and biotechnology firms, universities and other research institutions and government agencies that are developing and commercializing pharmaceutical products. Our key competitors focused on developing therapies in various indications, include among others, Pfizer, Freeline Therapeutics, Intellia Therapeutics, Sangamo Biosciences, Voyager Therapeutics, Passage Bio, Roche, PTC Therapeutics, Prilenia Therapeutics, CombiGene, Caritas Therapeutics, Alnylam, Wave Life Sciences, Bayer AG, Amicus Therapeutics and 4D Molecular Therapeutics.

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. A competitor approval may also prevent us from entering the market if the competitor receives any regulatory exclusivities that block our product candidates. 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 sales. From time to time, we publicly announce the expected timing of some of these milestones. All 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

We rely, and expect to continue to rely, on third parties to conduct, supervise, and monitor our preclinical studies and clinical trials, and those third parties may not perform satisfactorily, including failing to meet deadlines for the completion of such trials or failing to comply with regulatory requirements.

We rely on third parties, study sites, and others to conduct, supervise, and monitor our preclinical and clinical trials for our product candidates and do not currently plan to independently conduct clinical or preclinical trials of any other potential product candidates. We expect to continue to rely on third parties, such as CROs, clinical data management organizations, medical and scientific institutions, and clinical and preclinical investigators, to conduct our preclinical studies and clinical trials.

While we have agreements governing the activities of such third parties, we have limited influence and control over their actual performance and activities. For instance, our third-party service providers are not our employees, and except for remedies available to us under our agreements with such third parties we cannot control whether or not they devote sufficient time and resources to our ongoing clinical, non-clinical, and preclinical programs. If these third parties do not successfully carry out their contractual duties, meet expected deadlines or conduct our preclinical studies or clinical trials in accordance with regulatory requirements or our stated protocols, if they need to be replaced or if the quality or accuracy of the data they obtain is compromised due to the failure to adhere to our protocols, regulatory requirements or for other reasons, our trials may be repeated, extended, delayed, or terminated, we may not be able to obtain, or may be delayed in obtaining, marketing approvals for our product candidates, we may not be able to, or may be delayed in our efforts to, successfully commercialize our product candidates, or we or they may be subject to regulatory enforcement actions. As a result, our results of operations and the commercial prospects for our product candidates would be harmed, our costs could increase and our ability to generate revenues could be delayed. To the extent we are unable to successfully identify and manage the performance of third-party service providers in the future, our business may be materially and adversely affected. Our third-party service providers may also have relationships with other entities, some of which may be our competitors, for whom they may also be conducting trials or other therapeutic development activities that could harm our competitive position.

Our reliance on these third parties for development activities will reduce our control over these activities. Nevertheless, we are responsible for ensuring that each of our studies is conducted in accordance with the applicable protocol, legal, regulatory, and scientific standards, and our reliance on third parties does not relieve us of our regulatory responsibilities. For example, we will remain responsible for ensuring that each of our trials is conducted in accordance with the general investigational plan and protocols for the trial. We must also ensure that our preclinical trials are conducted in accordance with GLPs, as appropriate. Moreover, the FDA and comparable foreign regulatory authorities require us to comply with GCPs for conducting, recording, and reporting the results of clinical trials to assure that data and reported results are credible and accurate and that the rights, integrity, and confidentiality of trial participants are protected. Regulatory authorities enforce these requirements through periodic inspections of trial sponsors, clinical and preclinical investigators, and trial sites. If we or any of our third-party service providers fail to comply with applicable GCPs or other regulatory requirements, we or they may be subject to enforcement or other legal actions, the data generated in our trials may be deemed unreliable and the FDA or comparable foreign regulatory authorities may require us to perform additional studies.

In addition, we will be required to report on certain financial interests of our third-party investigators if these relationships exceed certain financial thresholds or meet other criteria. The FDA or comparable foreign regulatory authorities may question the integrity of the data from those clinical trials conducted by investigators who may have conflicts of interest.

We cannot assure that upon inspection by a given regulatory authority, such regulatory authority will determine that any of our trials complies with the applicable regulatory requirements. In addition, our clinical trials must be conducted with product candidates that were produced under GMP conditions. Failure to comply with these regulations may require us to repeat clinical trials, which would delay the regulatory approval process. We also are required to register certain clinical trials and post the results of certain completed clinical trials on a government-sponsored database, ClinicalTrials.gov, within specified timeframes. Failure to do so can result in enforcement actions and adverse publicity.

Agreements with third parties conducting or otherwise assisting with our clinical or preclinical studies might terminate for a variety of reasons, including a failure to perform by the third parties. If any of our relationships with these third parties terminate, we may not be able to enter into arrangements with alternative providers or to do so on commercially reasonable terms. Switching or adding additional third parties involves additional costs and requires management time and focus. In addition, there is a natural transition period when a new third party commences work. As a result, if we need to enter into alternative arrangements, it could delay our product development activities and adversely affect our business. Though we carefully manage our relationships with our third parties, there can be no assurance that we will not encounter challenges or delays in the future or that these delays or challenges will not have a material adverse impact on our business, financial condition and prospects, and results of operations.

We also rely on other third parties to store and distribute our products for the clinical and preclinical trials that we conduct. Any performance failure on the part of our distributors could delay the development, marketing approval, or commercialization of our product candidates, producing additional losses and depriving us of potential product revenue.

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 collaborations 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 large part on our ability to obtain and maintain this protection in the U.S., 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. The patents we own currently are and may become subject to future patent opposition or similar proceedings. For example, we are currently defending a patent case in each of Canada, the United Kingdom, the Netherlands and the U.S. The latter defense is at the U.S. Court of Appeals for the Federal Circuit level. These oppositions and future patent oppositions 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 U.S. 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 U.S. 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 U.S. 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 condition, 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 on 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 a 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 U.S. 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.

In addition, legal proceedings relating to intellectual property claims, with or without merit, are unpredictable and generally expensive and time-consuming and is likely to divert significant resources from our core business, including distracting our technical and management personnel from their normal responsibilities. Furthermore, because of the substantial amount of discovery required in connection with intellectual property litigation, there is a risk that some of our confidential information could be compromised by disclosure during this type of litigation.

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 while 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 U.S. 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 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 U.S. 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 with whom they communicate, from using that technology or information to compete with us.

Intellectual property rights do not necessarily address all potential threats to our competitive advantage.

The degree of future protection afforded by our intellectual property rights is uncertain because intellectual property rights have limitations and may not adequately protect our business or permit us to maintain a competitive advantage. For example:

- others may be able to make gene therapy products that are similar to our product candidates or utilize similar gene therapy technology but that are not covered by the claims of the patents that we own or have licensed;
- we or our licensors or future collaborators might not have been the first to make the inventions covered issued patents or pending patent applications that we own or have licensed;
- we or our licensors or future collaborators might not have been the first to file patent applications covering certain of our inventions;
- others may independently develop similar or alternative technologies or duplicate any of our technologies without infringing our intellectual property rights;
- it is possible that our pending patent applications will not lead to issued patents;
- issued patents that we own or have licensed may be held invalid or unenforceable, as a result of legal challenges by our competitors;
- our competitors might conduct activities in countries where we do not have patent rights and then use the information learned from such activities to develop competitive products for sale in our major commercial markets;
- we may not develop additional proprietary technologies that are patentable; and
- the patents of others may have an adverse effect on our business.

Should any of these events occur, they could seriously harm our business.

Risks Related to Business Development

Our business development strategy may not produce the cash flows expected or could result in additional costs and challenges.

Any business development transaction could expose us to unknown liabilities and risks, and we may incur additional costs and expenses necessary to address an acquired company's failure to comply with laws and governmental rules and regulations. We could incur additional costs related to resources to align our business practices and operations. Moreover, we cannot assure that the anticipated benefits of any acquisition would be realized in a timely manner, if at all.

Risks Related to Pricing and Reimbursement

We, and our commercial partner, face uncertainty related to insurance coverage of, and pricing and reimbursement for HEMGENIX® and other 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 and negotiating or requiring payment of manufacturer rebates. 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.

Moreover, payment methodologies may be subject to changes in healthcare legislation and regulatory initiatives. For example, the Center for Medicare & Medicaid Innovation at the Centers for Medicare & Medicaid Services ("CMS") may develop new payment and delivery models, such as bundled payment models. In addition, recently there has been heightened governmental scrutiny over the manner in which manufacturers set prices for their marketed products, which has resulted in several U.S. Congressional inquiries and proposed and enacted federal and state legislation designed to, among other things, bring more transparency to drug pricing, reduce the cost of prescription drugs under government payor programs, and review the relationship between pricing and manufacturer patient assistance programs. Most recently, on August 16, 2022, the Inflation Reduction Act of 2022, or IRA, was signed into law. Among other things, the IRA requires manufacturers of certain drugs to engage in price negotiations with Medicare (with the maximum fair prices for the first year of the negotiation program being initially applicable in 2026), with prices that can be negotiated subject to a cap; imposes rebates for certain drugs under Medicare Part B and Medicare Part D to penalize price increases that outpace inflation (first due in 2023); and replaces the Part D coverage gap discount program with a new discounting program (beginning in 2025). We expect that additional U.S. federal healthcare reform measures will be adopted in the future, any of which could limit the amounts that the U.S. federal government will pay for healthcare products and services, which could result in reduced demand for our product candidates or additional pricing pressures and could seriously harm our business.

Individual states in the U.S. have also increasingly passed legislation and implemented regulations designed to control pharmaceutical and biological product pricing, including price or patient reimbursement constraints, discounts, restrictions on certain product access and marketing cost disclosure and transparency measures, and, in some cases, designed to encourage importation from other countries and bulk purchasing. Legally mandated price controls on payment amounts by third-party payors or other restrictions could seriously harm our business. In addition, regional healthcare authorities and individual hospitals are increasingly using bidding procedures to determine what pharmaceutical products and which suppliers will be included in their prescription drug healthcare programs. This could reduce the ultimate demand for our product candidates or put pressure on our product pricing. Furthermore, there has been increased interest by third-party payors and governmental authorities in reference pricing systems and publication of discounts and list prices. Prescription drugs and biological products that are in violation of these requirements will be included on a public list. These reforms could reduce the ultimate demand for our product candidates or put pressure on our product pricing and could seriously harm our business.

In the EU, similar political, economic, and regulatory developments may affect our ability to profitably commercialize our product candidates, if approved. In addition to continuing pressure on prices and cost containment measures, legislative developments at the EU or member state level may result in significant additional requirements or obstacles that may increase our operating costs. The delivery of healthcare in the EU, including the establishment and operation of health services and the pricing and reimbursement of medicines, is almost exclusively a matter for national, rather than EU, law and policy. National governments and health service providers have different priorities and approaches to the delivery of health care and the pricing and reimbursement of products in that context. In general, however, the healthcare budgetary constraints in most EU member states have resulted in restrictions on the pricing and reimbursement of medicines by relevant health service providers. Coupled with ever-increasing EU and national regulatory burdens on those wishing to develop and market products, this could prevent or delay marketing approval of our product candidates, restrict or regulate post-approval activities and affect our ability to commercialize our product candidates, if approved. In markets outside of the U.S. and EU, reimbursement and healthcare payment systems vary significantly by country, and many countries have instituted price ceilings on specific products and therapies.

We cannot predict the likelihood, nature or extent of government regulation that may arise from future legislation or administrative or judicial action in the U.S., the EU, or any other jurisdiction. If we or any third parties we may engage are slow or unable to adapt to changes in existing requirements or the adoption of new requirements or policies, or if we or such third parties are not able to maintain regulatory compliance, our product candidates may lose any regulatory approval that may have been obtained and we may not achieve or sustain profitability.

The pricing review period and pricing negotiations for new medicines take considerable time and have uncertain results. Pricing review and negotiation usually begin 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 could be adversely affected and our business could 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 our 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 could be adversely affected.

We also anticipate that many or all 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. Additionally, there may be situations in which our product candidates will need to be administered more than once, 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 had a loss in the six months ended June 30, 2023 and year ended December 31, 2022, incurred significant losses in previous years and expect to incur losses during the current and over the next several years and may never achieve or maintain profitability.

We had a net loss of $145.7 million in the six months ended June 30, 2023, and a net loss of $126.8 million in the full year 2022. As of June 30, 2023, we had an accumulated deficit of $727.6 million. In the past, we have financed our operations primarily through the sale of equity securities and convertible debt, venture loans, upfront payments from our collaboration partners and, to a lesser extent, subsidies and grants from governmental agencies and fees for services. We expect to finance our operations in 2023 and until the second quarter of 2026 primarily from our existing cash, cash equivalents, and cash resources. 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.

We anticipate that our expenses will increase for the foreseeable future and will include costs related to:

- advancing AMT-130 for our Huntington's disease gene therapy program into phase III clinical study;
- advancing our gene therapy programs for rTLE, SOD1-ALS and Fabry disease into Phase I/II clinical studies;
- potentially acquiring or in-licensing rights to new therapeutic targets, product candidates and technologies; and
- making potential future milestone payments related to the acquisition of uniQure France SAS, if any.

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 condition, results of operations and cash flows.

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

Adequate capital may not be available to us when needed or may not be available on acceptable terms. Our ability to obtain debt financing may be limited by covenants we have made under our 2023 Amended Facility with 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 could 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, 2023, we had $100.0 million of outstanding principal of borrowings under the 2023 Amended Facility, which we are required to repay in full in January 2027. 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 2023 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 employees, customers and third parties are subject to applicable laws and regulations, the non-compliance of any of which could have a material adverse effect on our business, financial condition, and results of operations.

Healthcare providers, physicians, other practitioners, 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 U.S. and international 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 could 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 from participation in government funded healthcare programs and the curtailment or restructuring of our operations.

Additionally, we are subject to various labor and employment laws and regulations. These laws and regulations relate to matters such as employment discrimination, wage and hour laws, requirements to provide meal and rest periods or other benefits, family leave mandates, employee and independent contractor classification rules, requirements regarding working conditions and accommodations to certain employees, citizenship or work authorization and related requirements, insurance and workers' compensation rules, healthcare laws, scheduling notification requirements and anti-discrimination and anti-harassment laws. Complying with these laws and regulations, including ongoing changes thereto, subjects us to substantial expense and non-compliance could expose us to significant liabilities. In particular, we are subject to allegations of Sarbanes-Oxley whistleblower retaliation and employment discrimination and retaliation, and we may in the future be subject to additional claims of non-compliance with similar or other Laws and regulations.

The costs associated with a violation of any of the foregoing 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 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, imposes 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 20.0 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.

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 or sites, or discontinuation of development programs;
- significant costs to defend the related litigation;
- substantial monetary awards to trial participants or patients;
- loss of revenue;
- initiation of investigations, and enforcement actions by regulators; and product recalls, withdrawals, revocation of approvals, or labeling, marketing, or promotional restrictions;
- reduced resources of our management to pursue our business strategy; and
- the inability to further develop or commercialize any products that we develop.

Depending upon the country where the clinical trial is conducted, we currently hold coverages ranging from EUR 500,000 to EUR 10,000,000 per occurrence. 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 U.S., 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, Congress subsequently has extended the period over which these reductions are in effect. While President Biden previously signed legislation temporarily to eliminate this reduction through the end of 2021, recent legislation will restart the reductions, which will thereafter remain in effect through 2031 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 additional downward pressure on pricing and the reimbursement our customers may receive for our products, and increased manufacturer rebates. 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 117th U.S. Congress and under the Biden 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 future growth may depend, in part, on our ability to penetrate foreign markets, where we would be subject to additional regulatory burdens and other risks and uncertainties.

Our future profitability may depend, in part, on our ability to commercialize current or future drug candidates in foreign markets for which we may rely on collaborations with third parties. We are not permitted to market or promote any of our drug candidates before we receive regulatory approval from the applicable regulatory authority in that foreign market. To obtain separate regulatory approval in many other countries we must comply with numerous and varying regulatory requirements of such countries regarding safety and efficacy and governing, among other things, clinical trials, manufacturing, commercial sales, pricing and distribution of our drug candidates, and we cannot predict success in these jurisdictions.

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 might increase our vulnerability to the above risk.

While we have experienced and addressed system failures, cyber-attacks, and security breaches in the past, 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. 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, data, 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.

Climate change, environmental, social and governance and sustainability initiatives may result in regulatory or structural industry changes that could require significant operational changes and expenditures, reduce demand for the Company's products and adversely affect our business, financial condition, and results of operations.

Greenhouse gases may have an adverse effect on global temperatures, weather patterns, and the frequency and severity of extreme weather and natural disasters. Such events could have a negative effect on our business. Concern over climate change may result in new or additional legislative and regulatory requirements to reduce or mitigate the effects of climate change on the environment, which could result in future tax, transportation cost, and utility increases. Moreover, natural disasters and extreme weather conditions may impact the productivity of our facilities, the operation of our supply chain, or consumer buying patterns. Any of these risks could have a material adverse effect on our business.

Climate change, environmental, social and governance and sustainability initiatives may result in regulatory or structural industry changes that could require significant operational changes and expenditures, reduce demand for the Company's products and adversely affect our business, financial condition, and results of operations.

Climate change, environmental, social and governance and sustainability are a growing global movement. Continuing political and social attention to these issues has resulted in both existing and pending international agreements and national, regional, and local legislation, regulatory measures, reporting obligations and policy changes. Also, there is increasing societal pressure in some of the areas where we operate, to limit greenhouse gas emissions as well as other global initiatives. These agreements and measures, including the Paris Climate Accord, may require, or could result in future legislation, regulatory measures or policy changes that would require operational changes, taxes, or purchases of emission credits to reduce emission of greenhouse gases from our operations, which may result in substantial capital expenditures.

Furthermore, increasing attention to climate change, sustainability and environmental, social and governance ("ESG") has resulted in governmental investigations, and public and private litigation, which could increase our costs or otherwise adversely affect our business or results of operations. In addition, organizations that provide information to investors on corporate governance and related matters have developed ratings processes for evaluating companies on their approach to ESG matters. Such ratings are used by some investors to inform their investment and voting decisions. Unfavorable ESG ratings may lead to increased negative investor sentiment toward us, which could have a negative impact on the price of our securities and our access to and costs of capital.

Any or all of these ESG and sustainability initiatives may result in significant operational changes and expenditures, reduced demand for our products, cause us reputational harm, and could materially adversely affect our business, financial condition, and results of operations.

Risks Related to Employee Matters and Managing Growth

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

Our future growth and success will depend in large part on our continued ability to attract, retain, manage, and motivate our employees. The loss of the services of any member of our senior management or the inability to hire or retain experienced management personnel could adversely affect our ability to execute our business plan and harm our operating results. 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.

The competition for qualified personnel in the pharmaceutical field is intense, and there is a limited pool of qualified potential employees to recruit. Due to this intense competition, we may be unable to continue to attract and retain the qualified personnel necessary for the development of our business or to recruit suitable replacement personnel. If we are unable to continue to attract and retain high quality personnel, our ability to pursue our business may be harmed and our growth strategy may be limited.

Additionally, we are reliant on our employees, contractors, consultants, vendors, and other parties with whom we have relationships to behave ethically and within the requirements of the law. The failure of any employee or other such third parties to act within the bounds of the applicable laws, regulations, agreements, codes and other requirements, or any misconduct or illegal actions or omissions by such persons, could materially damage our business.

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 27, 2023, 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 27, 2023, was $9.51 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 and market reaction to our interim data from clinical trials;
- public perception of gene therapy;
- interactions with the FDA on the design of our clinical trials and regulatory endpoints;
- regulatory delays and greater government regulation of potential products due to adverse events;
- regulatory or legal developments in the EU, the U.S., 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.

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 37.8% of our issued shares (including such shares to be issued in relation to exercisable options to purchase ordinary shares) as of June 30, 2023. 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 of 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 those 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.

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 consequences 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 and 2022 but not for 2017 through 2021. A corporation organized outside the U.S. 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, 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 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 U.S. As a result, it may not be possible for shareholders to effect service of process within the U.S. 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 U.S. 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 U.S. brought in a court of competent jurisdiction in the Netherlands.

The U.S. 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 U.S., 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 U.S. 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 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 actions 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

During the three months ended June 30, 2023, no director or officer of the Company adopted or terminated a "Rule 10b5-1 trading arrangement" or "non-Rule 10b5-1 trading arrangement," as each term is defined in Item 408(a) of Regulation S-K.

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

10.1*†	Amendment No. 1 to Third Amended and Restated Loan and Security Agreement, dated as of May 12, 2023, by and among uniQure biopharma, B.V., uniQure, Inc., uniQure IP B.V., uniQure N.V., and Hercules Capital, Inc.
10.2*†	Royalty Purchase Agreement, dated May 12, 2023, by and between uniQure biopharma B.V. and HemB SPV, L.P.
31.1*	Certification of Chief Executive Officer pursuant to Rule 13a-14(a) or 15d-14(a) of the Securities and Exchange Act of 1934, as amended, as adopted pursuant to Section 302 of the Sarbanes-Oxley Act of 2002.
31.2*	Certification of Chief Financial Officer pursuant to Rule 13a-14(a) or 15d-14(a) of the Securities and Exchange Act of 1934, as amended, as adopted pursuant to Section 302 of the Sarbanes-Oxley Act of 2002.
32.1±	Certification of Principal Executive Officer and Principal Financial Officer pursuant to 18 U.S.C. Section 1350, as adopted pursuant to Section 906 of the Sarbanes-Oxley Act of 2002.
101*	The following financial information from our Quarterly Report on Form 10-Q for the period ended June 30, 2023, filed with the Securities and Exchange Commission on August 1, 2023, 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, 2023, filed with the Securities and Exchange Commission on August 1, 2023, is formatted in Inline Extensible Business Reporting Language ("iXBRL")

† Pursuant to Item 601(b)(10)(iv) of Regulation S-K promulgated by the SEC, certain portions of this exhibit have been omitted. The Company hereby agrees to furnish supplementally to the SEC, upon its request, an unredacted copy of this exhibit.

* Filed herewith.

± Furnished herewith.

SIGNATURE

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

UNIQURE N.V.

By: /s/ Matthew Kapusta

Matthew Kapusta
Chief Executive Officer
(Principal Executive Officer)

By: /s/ Christian Klemt

Christian Klemt
Chief Financial Officer
(Principal Financial Officer)

Dated August 1, 2023

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

AMENDMENT NO. 1 TO THIRD AMENDED AND RESTATED LOAN AND SECURITY AGREEMENT

This **AMENDMENT NO. 1 TO THIRD AMENDED AND RESTATED LOAN AND SECURITY AGREEMENT** (this "**Amendment**"), is dated as of May 12, 2023 and is entered into by and among (a) (i) **UNIQURE BIOPHARMA B.V.**, a private limited liability company incorporated and existing under the laws of the Netherlands, having its corporate seat at Amsterdam, the Netherlands and registered at the trade register of the Chamber of Commerce for Amsterdam under number 34275365 ("**uniQure Bio**"), (ii) **UNIQURE, INC.**, a Delaware corporation ("**US Borrower**" and together with uniQure Bio hereinafter collectively referred to as "**Borrower**"), (iii) **UNIQURE IP B.V.**, a private limited liability company incorporated and existing under the laws of the Netherlands, having its corporate seat at Amsterdam, the Netherlands and registered at the trade register of the Chamber of Commerce for Amsterdam under number 34275369 ("**uniQure IP**"), and (iv) **UNIQURE N.V.** (formerly uniQure B.V.), a public limited company incorporated and existing under the laws of the Netherlands, having its corporate seat at Amsterdam, the Netherlands and registered at the trade register of the Chamber of Commerce for Amsterdam under number 54385229 ("**uniQure Holdings**" and, together with Borrower and uniQure IP, the "**Obligors**"), (b) **HERCULES CAPITAL, INC.**, a Maryland corporation in its capacity as administrative agent and collateral agent for itself and the Lender (as defined herein) (in such capacity, "**Agent**"), and (c) the several banks and other financial institutions or entities from time to time parties to the Loan Agreement (collectively, referred to as "**Lender**"). Capitalized terms used herein without definition shall have the same meanings given them in the Amended Loan Agreement (as defined below).

RECITALS

A. **WHEREAS**, Obligors, Agent and Lender have entered into that certain Third Amended and Restated Loan and Security Agreement, dated as of December 15, 2021 (as amended, restated, amended and restated, supplemented or otherwise modified from time to time, the "**Loan Agreement**"), pursuant to which Lender has agreed to extend and make available to Borrower certain advances of money;

B. **WHEREAS**, uniQure Bio has entered into that certain Royalty Purchase Agreement (the "**HCRx Agreement**"), dated as of May 12, 2023, by and among uniQure Bio, as seller, and HemB SPV, L.P. ("**HCRx**"), as purchaser, pursuant to which HCRx will acquire rights to certain royalty interest payments under the CSL Licenses from uniQure Bio (the transactions contemplated under the HCRx Agreement, the "**HCRx Transaction**");

C. **WHEREAS**, Borrower has requested that Agent and Lender agree to amend the Loan Agreement to (i) extend each of the Amortization Date and Term Loan Maturity Date to January 5, 2027 and (ii) amend certain other terms of the Loan Agreement; and

D. **WHEREAS**, Obligors, Agent and Lender have agreed to amend the Loan Agreement, upon the terms and conditions more fully set forth herein.

AGREEMENT

NOW THEREFORE, in consideration of the foregoing Recitals and intending to be legally bound, the parties hereto agree as follows:

1. HCRX TRANSACTION. The Obligors, Agent and Lender acknowledge and agree that the HCRx Transaction is a Permitted Royalty Transaction pursuant to the terms of the Loan Agreement [*]. Without limiting the generality of the foregoing, Agent and Lender (i) consent to the arrangements set forth in the HCRx Agreement [*]. Notwithstanding the foregoing, uniQure Bio hereby agrees to comply with clause (viii) of the definition of Permitted Royalty Transaction at the time of each payment to it under the HCRx Agreement, and the failure to comply with such requirements shall be an Event of Default under the Loan Agreement. Concurrently with the closing of the HCRx Transaction and the funding of the initial payment in accordance with the terms of the HCRx Agreement, (i) Agent hereby agrees to execute and deliver to Borrower a Partial Release Agreement in the form attached hereto as Exhibit A, and (ii) Agent and Lender consent in writing to the release pursuant to such Partial Release Agreement of all security rights created by uniQure Bio over the Assigned Assets (as defined therein) under or pursuant to the Loan Documents, including the undisclosed pledge of receivables dated June 13, 2013 between, amongst others, the uniQure Bio as pledgor and the Agent as pledgee.

2. AMENDMENTS. In each case subject to the satisfaction of the conditions specified in Section 5 hereof:

2.1. Section 1.1 of the Loan Agreement is hereby amended by inserting the following definitions in appropriate alphabetical order therein:

" "**Amendment Effective Date**" means May 12, 2023."

" "**Back End Fee**" shall have the meaning assigned to such term in Section 2.6(b)."

2.2. The defined term "Amortization Date" set forth in Section 1.1 of the Loan Agreement is hereby amended and restated in its entirety as follows:

" "**Amortization Date**" means the Term Loan Maturity Date."

2.3. The defined term "Term Loan Maturity Date" set forth in Section 1.1 of the Loan Agreement is hereby amended and restated in its entirety as follows:

" "**Term Loan Maturity Date**" means January 5, 2027."

2.4. Section 2.6 of the Loan Agreement is hereby amended and restated in its entirety as follows:

"Additional End of Term Charges.

(a) On the earliest to occur of (i) December 1, 2025, (ii) the date that Borrower prepays the outstanding Secured Obligations in full, or (iii) the date that the Secured Obligations become due and payable, Borrower shall pay Lender an additional charge equal to $[*] (the "**Term Loan End of Term Charge**"). Notwithstanding the required payment date of such Term Loan End of Term Charge, it shall be deemed earned by Lender as of the Restatement Date.

(b) On the earliest to occur of (i) January 5, 2027, (ii) the date that Borrower prepays the outstanding Secured Obligations in full, or (iii) the date that the Secured

Obligations become due and payable, Borrower shall pay Lender an additional charge equal to $[*] (the "**Back End Fee**" and, together with the 2018 End of Term Charge, the 2021 End of Term Charge and the Term Loan End of Term Charge, the "**End of Term Charge**"). Notwithstanding the required payment date of such Back End Fee, it shall be deemed earned by Lender as of the Amendment Effective Date."

2.5. Each reference in the Loan Agreement to "this Agreement" and the words "hereof," "herein," "hereunder," or words of like import, shall mean and be a reference to the Loan Agreement as amended by this Amendment (the "**Amended Loan Agreement**").

3. BORROWER'S REPRESENTATIONS AND WARRANTIES. Borrower represents and warrants that:

3.1. Immediately upon giving effect to this Amendment (i) the representations and warranties contained in the Loan Documents are true, accurate and complete in all material respects except to the extent such representations and warranties relate to an earlier date, in which case they are true and correct in all material respects as of such date (in all cases without duplication of any standard(s) of materiality contained in the Loan Documents as to such representations and warranties) and (ii) no Event of Default has occurred and is continuing with respect to which Borrower has not been notified in writing by Agent or Lender;

3.2. Borrower has the corporate or other applicable company power and authority to execute and deliver this Amendment and to perform its obligations under the Amended Loan Agreement;

3.3. [Reserved.]

3.4. The execution and delivery by Borrower of this Amendment and the performance by Borrower of its obligations under the Amended Loan Agreement have been duly authorized by all necessary corporate or other applicable company action on the part of Borrower;

3.5. Subject to any matters which are set out as qualifications or reservations as to matters of law of general application in the legal opinions delivered to the Lender pursuant to the Loan Agreement, this Amendment has been duly executed and delivered by Borrower and is the binding obligation of Borrower, enforceable against it in accordance with its terms, except as such enforceability may be limited by bankruptcy, insolvency, reorganization, liquidation, moratorium or other similar laws of general application and equitable principles relating to or affecting creditors' rights; and

3.6. As of the date hereof, it has no defenses against the obligations to pay any amounts under the Secured Obligations. Borrower acknowledges that each of Agent and Lender has acted in good faith and has conducted in a commercially reasonable manner its relationships with Borrower in connection with this Amendment and in connection with the Loan Documents.

Borrower understands and acknowledges that each of Agent and Lender is entering into this Amendment in reliance upon, and in partial consideration for, the above representations and warranties, and agrees that such reliance is reasonable and appropriate.

4. LIMITATION. The amendments set forth in this Amendment shall be limited precisely as written and shall not be deemed (a) to be a waiver or modification of any other term or condition of the Loan Agreement or of any other instrument or agreement referred to therein or to prejudice any right or remedy which Agent and/or Lender may now have or may have in the future under or in connection with the Amended Loan Agreement or any instrument or agreement referred to therein; or (b) to be a consent to any future amendment or modification or waiver to any instrument or agreement the execution and delivery of which is consented to hereby, or to any waiver of any of the provisions thereof. Except as expressly amended hereby, the Loan Agreement shall continue in full force and effect.

5. EFFECTIVENESS. This Amendment shall become effective upon the satisfaction of all the following conditions precedent (the date of satisfaction of all such conditions precedent, the "**First Amendment Effective Date**"):

5.1. Amendment. Obligors, Agent and Lender shall have duly executed and delivered this Amendment to Lender.

5.2. Payment of Lender Expenses. Subject to Section 6 below, Borrower shall have paid all reasonable and invoiced Lender expenses incurred through the date of this Amendment in an amount not to exceed $50,000.

6. CONDITIONS SUBSEQUENT.

6.1. Payment of Lender Expenses. Within five (5) Business Days after the First Amendment Effective Date (or such later date as may be agreed by the Agent in its sole discretion), Borrower shall have paid all reasonable and invoiced attorneys' fees and reasonable expenses incurred in connection with the documentation, negotiation, execution and closing of this Amendment and through the date of this Amendment in an amount not to exceed $50,000.

The failure to comply with any of the covenants set forth in this Section 6 within the applicable time frame set forth above shall constitute an immediate Event of Default.

7. RELEASE. In consideration of the agreements of Agent and each Lender contained herein and for other good and valuable consideration, the receipt and sufficiency of which are hereby acknowledged, Borrower, on behalf of itself and its successors, assigns, and other legal representatives, hereby to the extent possible under applicable law fully, absolutely, unconditionally and irrevocably releases, remises and forever discharges Agent and each Lender, and its successors and assigns, and its present and former shareholders, affiliates, subsidiaries, divisions, predecessors, directors, officers, attorneys, employees, agents and other representatives (Agent, Lenders and all such other persons being hereinafter referred to collectively as the "**Releasees**" and individually as a "**Releasee**"), of and from all demands, actions, causes of action, suits, covenants, contracts, controversies, agreements, promises, sums of money, accounts, bills, reckonings, damages and any and all other claims, counterclaims, defenses, rights of set-off, demands and liabilities whatsoever of every name and nature, known or unknown, suspected or unsuspected, both at law and in equity, which Borrower, or any of its successors, assigns, or other legal representatives may now or hereafter own, hold, have or claim to have against the Releasees or any of them for, upon, or by reason of any circumstance, action, cause or thing whatsoever which arises at any time on or prior to the day and date of this Amendment, for or on account of, or in relation to, or in any way in connection with the Loan Agreement, or any of the other Loan Documents or transactions thereunder or related thereto. Borrower understands, acknowledges and agrees that the release set forth above may be pleaded as a full and complete defense and may be used as a basis for an injunction against any action, suit or other proceeding which may be instituted, prosecuted or attempted in breach of the provisions of such release. Borrower agrees that no fact, event, circumstance, evidence or transaction which could now be

asserted or which may hereafter be discovered shall affect in any manner the final, absolute and unconditional nature of the release set forth above.

8. COUNTERPARTS. This Amendment may be signed in any number of counterparts, and by different parties hereto in separate counterparts, with the same effect as if the signatures to each such counterpart were upon a single instrument. All counterparts shall be deemed an original of this Amendment. This Amendment may be executed by facsimile, portable document format (.pdf) or similar technology signature, and such signature shall constitute an original for all purposes.

9. INCORPORATION BY REFERENCE. The provisions of Section 10 (Miscellaneous) of the Loan Agreement shall be deemed incorporated herein by reference, *mutatis mutandis*.

10. LOAN DOCUMENTS. This Amendment shall constitute a Loan Document.

[*Remainder of this page intentionally left blank*]

IN WITNESS WHEREOF, the parties have duly authorized and caused this Amendment to be executed as of the date first written above.

BORROWERS:

UNIQURE BIOPHARMA B.V.

Signature: /s/ Christian Klemt
Print Name: Christian Klemt
Title: Chief Financial Officer, Director

UNIQURE, INC.

Signature: /s/ Matt Kapusta
Print Name: Matt Kapusta
Title: Chief Executive Officer

OBLIGORS:

UNIQURE N.V. (formerly uniQure B.V.)

Signature: /s/ Matt Kapusta
Print Name: Matt Kapusta
Title: Chief Executive Officer

UNIQURE IP B.V.

Signature: /s/ Matt Kapusta
Print Name: Matt Kapusta
Title: Chief Executive Officer

Accepted in Palo Alto, California:

AGENT:

HERCULES CAPITAL, INC.

By: /s/ [*]
Name: [*]
Its: [*]

LENDER:

HERCULES CAPITAL, INC.

By: /s/ [*]
Name: [*]
Its: [*]

HERCULES FUNDING IV, LLC

By: /s/ [*]
Name: [*]
Its: [*]

HERCULES PRIVATE CREDIT FUND 1 L.P.

By: Hercules Adviser LLC, its Investment Adviser

By: /s/ [*]
Name: [*]
Its: [*]

HERCULES PRIVATE GLOBAL VENTURE GROWTH FUND I L.P.

By: Hercules Adviser LLC, its Investment Adviser

By: /s/ [*]
Name: [*]
Its: [*]

EXHIBIT A

Form of Partial Release Agreement

[*]

Exhibit 10.2

ROYALTY PURCHASE AGREEMENT[1]

dated as of May 12, 2023

between

UNIQURE BIOPHARMA BV

and

HEMB SPV, L.P.

[1] CERTAIN IDENTIFIED INFORMATION HAS BEEN EXCLUDED (INDICATED BY: [***]) FROM THE EXHIBIT BECAUSE IT IS BOTH (I) NOT MATERIAL AND (II) THE TYPE OF INFORMATION THAT THE REGISTRANT CUSTOMARILY AND ACTUALLY TREATS AS PRIVATE OR CONFIDENTIAL.

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ROYALTY PURCHASE AGREEMENT

This ROYALTY PURCHASE AGREEMENT (this "**Agreement**") dated as of May 12, 2023 (the "**Execution Date**") is between uniQure biopharma BV ("**Seller**"), a corporation organized under the laws of the Netherlands, and **HemB SPV, L.P.**, a Delaware limited partnership ("**Purchaser**" and collectively with Seller, the "**Parties**" and each, a "**Party**").

RECITALS

WHEREAS, Seller is a party to the License Agreement (as defined below); and

WHEREAS, Seller desires to sell, transfer, assign and convey to Purchaser, and Purchaser desires to purchase, acquire and accept from Seller, Seller's rights, title, and interests in and to the Purchased Receivables, upon and subject to the terms and conditions set forth in this Agreement;

NOW, THEREFORE, in consideration of the premises and the mutual agreements, representations, and warranties set forth herein and of other good and valuable consideration, the receipt and adequacy of which are hereby acknowledged, the Parties covenant and agree as follows:

ARTICLE 1
DEFINED TERMS

The following terms, as used herein, will have the following respective meanings:

1.1 "**Accounting Firm**" has the meaning set forth in Section 5.2 (Inspections and Audits of Seller).

1.2 "**Affiliate**" means, with respect to any particular Person, any other Person directly or indirectly controlling, controlled by or under common control with such particular Person. For purposes of this definition, "**control**" means (a) in the case of corporate entities, direct or indirect ownership of at least [***]% of the stock or shares having the right to vote for the election of directors, and (b) in the case of non-corporate entities, direct or indirect ownership of at least [***]% of the equity interest with the power to direct the management and policies of such non-corporate entities.

1.3 "**Agreement**" has the meaning set forth in the preamble.

1.4 "**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.

1.5 "**Back-Up Security Interest**" has the meaning set forth in Section 2.1.2 (Financing Statements).

1.6 "**Bankruptcy Law**" means Title 11 of the United States Code entitled "Bankruptcy" and all other liquidation, conservatorship, bankruptcy, assignment for the benefit of creditors, moratorium, rearrangement, receivership, insolvency, reorganization, or similar debtor

relief laws of any jurisdiction in the United States or other applicable jurisdictions (domestic or foreign) from time to time in effect affecting the rights of creditors generally.

1.7 "**Bill of Sale**" means that certain bill of sale dated as of the Closing Date executed by Seller and Purchaser substantially in the form of Exhibit A.

1.8 "**BLA**" means a Biologics License Application, as defined in the United States Public Health Service Act (42 U.S.C. § 262), and applicable regulations promulgated thereunder by the FDA, or any equivalent application that replaces such application and is the relevant equivalent to the foregoing, or any analogous application or submission with any Regulatory Authority outside of the United States.

1.9 "**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.

1.10 "**Business Representations**" has the meaning set forth in Section 9.1 (Survival of Representations and Warranties).

1.11 "**Cap Amount**" means, as of the date of determination thereof during the Term, the applicable Cap Multiplier *multiplied by* the Purchase Price paid to Seller as of such date, *minus* the Cap Payment (if applicable).

1.12 "**Cap Date**" means the first date on which the Total Net Amount as of such date equals the Cap Amount.

1.13 "**Cap Multiplier**" means, as of the date of determination thereof during the Term, (a) if such date is on or prior to June 30, 2032, then 185%, and (b) if such date is after June 30, 2032, then 225%.

1.14 "**Cap Payment**" has the meaning set forth in Section 2.5 (Cap Payment Option).

1.15 "**Closing**" has the meaning set forth in Section 6.1 (Closing).

1.16 "**Closing Date**" has the meaning set forth in Section 6.1 (Closing).

1.17 "**Code**" means the U.S. Internal Revenue Code of 1986, as amended from time to time.

1.18 "**Confidential Information**" means, collectively, all proprietary and non-public information (whether written or oral, or in electronic or other form, and whether furnished on or after the Execution Date) concerning, or relating in any way, directly or indirectly, to Seller, this Agreement, the License Agreement, Royalty Payments, or the Receivables, including (a) any license, sublicense, or other agreements involving or relating in any way, directly or indirectly, to the Receivables or the intellectual property, materials, or products (including the Product) giving rise to the Receivables, whether or not such licenses, sublicenses, or other agreements currently exist, are executed after the Execution Date, have been or are amended, or have been previously terminated, and including all terms and conditions hereof and thereof and the identities of the parties thereto, (b) any Reports, the

License Agreement, the Receivables, or the intellectual property, materials, or products giving rise to the Receivables, and including reports, data, information, materials, notices, correspondence, or documents of any kind delivered pursuant to or under this Agreement or any of the other agreements referred to in clause (a), and (c) any inventions, devices, improvements, formulations, discoveries, compositions, ingredients, patents, patent applications, know-how, processes, trial results, research, developments, or any other intellectual property, trade secrets, or information involving or relating in any way, directly or indirectly, to the Receivables or the materials or products (including the Product) giving rise to the Receivables. Notwithstanding the foregoing, "**Confidential Information**" will not include any information that (i) was known by Purchaser at the time such information was disclosed to Purchaser, its Affiliates or its or its Affiliates' Representatives in accordance herewith or in accordance with the Confidentiality Agreement, as evidenced by its written records or other competent evidence; (ii) was or becomes part of the public domain (other than as a result of a disclosure by Purchaser, its Affiliates, or its or its Affiliates' Representatives in violation of such disclosing Person's confidentiality obligations); (iii) becomes known to Purchaser on a non-confidential basis from a source other than Seller, its Affiliates, or its and its Affiliates' Representatives (and without any breach of this Agreement or the Confidentiality Agreement by Purchaser, its Affiliates or its or its Affiliates' Representatives); provided that such source (A) had the right to disclose such information to Purchaser (without breaching any legal, contractual or fiduciary obligation to Seller or any of its Affiliates) and (B) did not obtain such information directly or indirectly from, or on behalf of, Seller, its Affiliates or its or its Affiliates' Representatives; or (iv) is or has been independently developed by Purchaser, its Affiliates, or its or its Affiliates' Representatives without use of or reference to the Confidential Information.

1.19 "**Confidentiality Agreement**" means that certain Confidential Disclosure Agreement, between Seller and Purchaser, dated as of December 12, 2022.

1.20 "**Confidentiality Breach**" means, with respect to the disclosure of any notice, demand, certificate, correspondence, report, or other communication under the License Agreement (or any information contained therein) to Purchaser, that such disclosure would, based upon the written guidance of nationally recognized outside legal counsel, constitute a breach by Seller of its confidentiality obligations under the License Agreement.

1.21 "**Deposit Account Control Agreement**" means the deposit account control agreement (or equivalent thereto in the Netherlands) entered into by the Depositary Bank, Purchaser, and Seller, as amended, supplemented or otherwise modified from time to time and any replacements thereof, in form and substance reasonably acceptable to Purchaser and Seller.

1.22 "**Depositary Bank**" means such bank or other financial institution approved by Purchaser and Seller located in the Netherlands or, if a deposit account with protections for Purchaser similar to those afforded by a "deposit account" (as defined in Article 9 of the UCC), then in the U.S., including any successor Depositary Bank appointed pursuant to Section 2.9 (Lockbox Account).

1.23 "**Disputes**" has the meaning set forth in Section 3.15.7 (Intellectual Property Matters)

1.24 "**Dollar**" or the sign "**$**" means United States dollars.

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

1.26 "**E.U.**" means those countries within the economic, scientific, and political organization of member states of the European Union, as it may be constituted from time to time, relying upon the central regulatory approval of AMT-061 by the EMA.

1.27 "**Excluded Assets**" means, individually and collectively: (a) the Seller IP Assets, (b) any Royalty Payments to the extent attributable to Net Sales with respect to Licensed Products prior to [***] or after the Cap Date; (c) any Royalty Payments to the extent in excess of the Purchaser Percentage Interest; and (d) any and all other rights of Seller, including to payment, compensation, or consideration, under or in respect of the License Agreement (other than (i) rights, payments, and amounts paid or payable under the License Agreement in lieu of Royalty Payments (*e.g.*, Proceeds pursuant to any enforcement of the Listed Patent Rights in accordance with Section 5.11.4), (ii) the Purchased Receivables, and (iii) any Proceeds payable to Seller as a result of actions taken by Seller in accordance with Section 5.10 (License Agreement) or Section 5.11.4 (Listed Patent Rights) hereof that are to be shared with Purchaser in accordance with such Section).

1.28 "**Excluded Liabilities and Obligations**" has the meaning set forth in 2.3 (No Assumed Obligations).

1.29 "**Execution Date**" has the meaning set forth in the preamble.

1.30 "**Existing In-License Payments**" means the portion of each Royalty Payment due from Seller to any Third Party under an Existing In-License in respect of Net Sales of the Product.

1.31 "**Existing In-Licenses**" means the agreements set forth on Exhibit C.

1.32 "**Exploitation**" means the manufacture, use, sale, offer for sale (including marketing and promotion), importation, distribution, or other commercialization.

1.33 "**FDA**" means the United States Food and Drug Administration or any successor entity thereto.

1.34 "**Financing Statements**" has the meaning set forth in Section 2.1.2 (Purchase and Sale).

1.35 "**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.

1.36 "**Indemnification Cap**" has the meaning set forth in Section 7.4 (Limitations).

1.37 "**Indemnified Party**" means a Seller Indemnified Party or Purchaser Indemnified Party, as applicable.

1.38 "**Indemnified Tax**" means any non-U.S. withholding Tax (other than a Purchaser Connection Tax) withheld by or on behalf of Seller in respect of any payments made by or behalf of Seller to Purchaser or any permitted assignee under this Agreement; provided that Purchaser or its permitted assignee, as applicable, is a U.S. Person at the time such Tax is withheld. For the avoidance of doubt, if Purchaser assigns its rights under this Agreement to a permitted assignee, any non-U.S. withholding Tax imposed on any consideration paid by the permitted assignee to Purchaser for such assignment shall not be considered an Indemnified Tax (*i.e.*, no such consideration payable by the permitted assignee shall be considered paid by or on behalf of the Seller for purposes of this definition).

1.39 "**Insolvency Event**" means the occurrence of any of the following with respect to Seller:

(a) (i) an involuntary proceeding is commenced or an involuntary petition is filed in a court of competent jurisdiction seeking (A) relief in respect of Seller, or of a material part of the Purchased Receivables, under any Bankruptcy Law now or hereafter in effect, (B) the appointment of a receiver, trustee, custodian, sequestrator, conservator, or similar official for Seller or for a material part of the Purchased Receivables, or (C) the winding-up or liquidation of Seller, in each case, which proceeding or petition continues undismissed for 90 days or (ii) an order of a court of competent jurisdiction approving or ordering any of the foregoing is entered;

(b) Seller (i) voluntarily commences any proceeding or files any petition seeking relief under any Bankruptcy Law now or hereafter in effect, (ii) applies for the appointment of a receiver, trustee, custodian, sequestrator, conservator, or similar official for Seller or for a material part of the Purchased receivables, (iii) consents to the entry of an order of a court of competent jurisdiction described in clause (a)(ii) of this definition, (iv) makes a general assignment for the benefit of creditors, or (v) winds up or liquidates (except as permitted under this Agreement); or

(c) Seller takes any action authorizing its consent to, approval of, or acquiescence in, any of the acts set forth in clause (a) or (b) of this definition.

1.40 "**Instruction to Payor**" has the meaning set forth in Section 2.9.1 (Lockbox Account).

1.41 "**Judgment**" means any judgment, order, writ, injunction, citation, award, or decree of any nature.

1.42 "**Knowledge**" means, with respect to Seller, the current actual knowledge of the Chief Executive Officer, Chief Financial Officer, Chief Legal Officer, Vice President, Intellectual Property, and President, Research & Development.

1.43 "**License Agreement**" means that certain commercialization and license agreement dated as of June 24, 2020, between Seller and Licensee, as amended or otherwise modified from time to time.

1.44 "**Licensed Product**" has the meaning set forth in the License Agreement as of the Execution Date.

1.45 "**Licensee**" means CSL Behring LLC, a Delaware limited liability company.

1.46 "**Lien**" means any security interest, mortgage, pledge, hypothecation, assignment, deposit arrangement, encumbrance, lien (statutory or otherwise), charge against or interest in property or other priority or preferential arrangement in the nature of a security interest, in each case, to secure payment of a debt or performance of an obligation

1.47 "**Listed Patent Rights**" means those Patent Rights set forth on Exhibit B, including the following for each: (a) the application number; (b) the patent or registration number, if any; (c) the country or other jurisdiction where the Patent Right was issued, registered, or filed; and (d) the registered owner thereof.

1.48 "**Lockbox Account**" means the "deposit account" (as defined in Article 9 of the UCC, or equivalent account in the Netherlands), investment account, or other account in which funds are held or invested to or for the credit or account of Purchaser, established and maintained at any Depositary Bank solely for the purpose of receiving remittance of the Purchased Receivables and disbursement thereof as provided herein, and any successor Lockbox Account entered into in accordance with Section 2.9 (Lockbox Account).

1.49 "**Loss**" means any loss, liability, cost, expense (including reasonable costs of investigation and defense and reasonable attorneys' fees and expenses), charge, fine, penalty, obligation, Judgment, award, assessment, claim, or cause of action.

1.50 "**Marketing Approval**" means, a BLA approved by the FDA, a Marketing Authorization Application approved by the EMA under the centralized European procedure, or any corresponding non-U.S. or non-EMA application, registration or certification, as applicable, necessary to market the Product approved by the corresponding Regulatory Authority in the Territory, as applicable, including pricing and reimbursement approvals where required.

1.51 "**Material Adverse Effect**" means any one or more of: (a) a material adverse effect on the right or ability of Seller to consummate the transactions contemplated by the Transaction Documents and perform its obligations under the Transaction Documents, (b) a material adverse effect on the validity or enforceability of the Transaction Documents against Seller or the rights of Purchaser thereunder, (c) a material adverse effect on the rights of Seller under the License Agreement, or (d) an adverse effect on the timing, duration, or amount of the Purchased Receivables.

1.52 "**Milestone Event**" has the meaning set forth in Section 2.2 (Purchase Price).

1.53 "**Milestone Payment**" has the meaning set forth in Section 2.2 (Purchase Price).

1.54 "**Net Sales**" has the meaning set forth in the License Agreement as of the Execution Date.

1.55 "**Non-Warranting Parties**" has the meaning set forth in 9.5.1 (No Personal Liability).

1.56 "**Patent Office**" means the applicable patent office, including the United States Patent and Trademark Office and any comparable foreign patent office.

1.57 "**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.

1.58 "**Payment Direction and Confidentiality Letter**" means that certain Payment Direction and Confidentiality Letter dated as of the Closing Date executed by Seller and Purchaser in a form reasonably acceptable to Seller and Purchaser.

1.59 "**Percentage Interest**" means, with respect to Purchaser, [***]% *minus* the Seller Percentage Interest and, with respect to Seller, the Seller Percentage Interest.

1.60 "**Permitted Liens**" means the following:

(a) statutory or common law Liens of landlords and Liens of carriers, warehousemen, distributors, mechanics, materialmen, and suppliers and other Liens imposed by law or pursuant to customary reservations or retentions of title arising in the ordinary course of business;

(b) Liens created in favor of Purchaser pursuant to this Agreement;

(c) servitudes, easements, rights of way, restrictions, and other similar encumbrances on real property imposed by any law and Liens consisting of zoning or building restrictions, easements, licenses, restrictions on the use of property, or minor imperfections in title thereto that, individually and in the aggregate, are not material, and that do not in any case materially detract from the value of the property subject thereto or interfere with the ordinary course of the business of Seller or any of its Affiliates;

(d) Liens arising out of conditional sale, title retention, consignment, or similar arrangements for the sale of goods entered into in the ordinary course of business;

(e) any interest or title of a lessor or sublessor under any lease or sublease entered into in the ordinary course of business; and

(f) Liens on property of the Seller existing on the Execution Date and listed on Schedule 1.60 of Exhibit C.

1.61 "**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.

1.62 "**Proceeds**" means any amounts actually recovered by Seller from any Person as a result of any settlement or resolution of any actions, suits, proceedings, claims, or disputes (a) relating to, and to the extent involving, the failure by Licensee to make any Royalty Payments, to the extent included in the Receivables or (b) pursuant to and under the conditions set forth in Section 12.5.4 (Recoveries) of the License Agreement.

1.63 "**Product**" means AMT-061, in any dosage strength, concentration, or formulation, marketed in the United States as HEMGENIX®, and any and all other Licensed Products.

1.64 "**Product Rights**" means any and all of the following, as they exist throughout the Territory: (a) Seller IP Assets, (b) regulatory filings, submissions and approvals, including Marketing Approvals, with or from any Regulatory Authorities with respect to the Product, (c) the Existing In-Licenses, and (d) the License Agreement.

1.65 "**Purchase Price**" has the meaning set forth in Section 2.2 (Purchase Price).

1.66 "**Purchased Receivable Payments**" means, for each calendar quarter during the Purchased Receivable Period in each country in the Territory, on a country-by-country basis, an amount payable to Purchaser equal to all aggregate Purchased Receivables in each country in the Territory during the Purchased Receivable Period in such country; provided that the Purchased Receivable Payments, in the aggregate, will not exceed the Cap Amount.

1.67 "**Purchased Receivable Period**" means the period beginning on the Royalty Commencement Date and ending on the earlier of (a) December 31, 2038 and (b) the Cap Date.

1.68 "**Purchased Receivable Report**" has the meaning set forth in Section 5.9.1.

1.69 "**Purchased Receivables**" means, on any date during the Purchased Receivable Period for a given country in the Territory, (a) the Net Sales of the Product in such country in the Territory, as reflected in the applicable Royalty Report for such date and included in the applicable Royalty Payment for such date, *plus* (b) any interest on any amounts referred to in the immediately foregoing clause (a) payable by Licensee and actually received by Seller pursuant to Section 8.11 (Late Payments) of the License Agreement, then *minus* (c) any Royalty Deductions actually taken in respect of such Net Sales of the Product in such country in the Territory, as reflected in the applicable Royalty Report and factored into the applicable Royalty Payment and then *multiplied by* (d) the Purchaser Percentage Interest on such date (which formula will be the amount equal to [((a) + (b)) – (c)] * (d)). Notwithstanding any provision to the contrary set forth in this Agreement, the Purchased Receivables exclude all Excluded Assets.

1.70 "**Purchaser**" has the meaning set forth in the preamble.

1.71 "**Purchaser Account**" has the meaning set forth in Section 5.9.2 (Payments on Account of the Purchased Receivables).

1.72 "**Purchaser Connection Tax**" means any Tax to the extent imposed by reason of (a) any connection of Purchaser with the jurisdiction of the applicable taxing authority other than a connection arising from this Agreement or any transactions contemplated hereunder of (b) any failure of Purchaser to timely provide any applicable documentation that is reasonably requested by the Seller and that Purchaser is legally eligible to provide.

1.73 "**Purchaser Expenses**" has the meaning set forth in Section 2.8 (Purchaser Expenses).

1.74 "**Purchaser Fundamental Representations**" has the meaning set forth in Section Article 9 (Survival of Representations and Warranties).

1.75 "**Purchaser Indemnified Party**" has the meaning set forth in Section 7.2 (Indemnification by Seller).

1.76 "**Purchaser Percentage Interest**" means (a) during the Purchased Receivable Period, [***]% and (b) after the Purchased Receivable Period, [***]%.

1.77 "**Rebate Payment**" has the meaning set forth in Section 2.6 (Purchaser Rebate).

1.78 "**Receivables**" means (a) each Royalty Payment to the extent attributable to Net Sales of the Product sold in a country in the Territory during the Purchased Receivable Period, and (b) any interest on any amounts referred to in the immediately foregoing clause (a) payable by Licensee and actually received by Seller pursuant to Section 8.11 (Late Payments) of the License Agreement.

1.79 "**Regulatory Authority**" means any national or supranational Governmental Authority, including the FDA, the EMA, or such equivalent regulatory authority, or any successor agency thereto, that has responsibility for granting a Marketing Approval.

1.80 "**Reports**" means any Royalty Reports, Purchased Receivable Reports, assignments, notices, requests, correspondence, or other information furnished pursuant to this Agreement (including Article 5 (Covenants)).

1.81 "**Representatives**" means, with respect to any Person, the directors, board members, managers, officers, employees, agents, limited partners, investors, or advisors (including attorneys, accountants, consultants, scientists, and financial advisors) of such Person.

1.82 "**Responsible Seller Party**" means any employee of Seller referred to in the definition of "Knowledge" and any successor to such employee.

1.83 "**Retained Receivables**" means, on any date during the Purchased Receivable Period in a country in the Territory, the Receivables in such country *minus* the Purchased Receivables in such country.

1.84 "**Royalty Commencement Date**" means, for all countries in the Territory, [***].

1.85 "**Royalty Deductions**" means any adjustments, modifications, offsets, credits, reductions, or deductions to the Royalty Payments made pursuant to Section 8.3.2 (Royalty Reductions) of the License Agreement.

1.86 "**Royalty Parties**" means Purchaser, Seller, and any other Person(s) to whom Seller may sell, transfer, assign, or convey any rights, title, or interests in or to all or a portion of the Retained Receivables; provided that, for the avoidance of doubt, the term "Royalty Parties" will not include (a) any Person that obtains an interest in all or a portion of the Retained Receivables solely by reason of such Person being an inventor of any of the intellectual property giving rise to the Receivables or (b) a Person (other than Seller) that acquires an interest in the Retained Receivables directly or indirectly from a Person described in clause (a) above.

1.87 "**Royalty Payment**" means each royalty payment payable by Licensee pursuant to Section 8.3 (Royalties) of the License Agreement in respect of Net Sales of the Licensed Product sold in a country in the Territory after giving effect to all applicable Royalty Deductions in such countries in the Territory.

1.88 "**Royalty Report**" means the reports required to be delivered by Licensee pursuant to Section 8.3.3 (Royalty Payments and Reports) of the License Agreement, to the extent such reports relate to a Royalty Payment in the Territory.

1.89 "**Safety Notices**" means any recalls, field notifications, market withdrawals, warnings, "dear doctor" letters, investigator notices, safety alerts, or other notices of action issued or instigated by Seller, any of its Affiliates, or any Regulatory Authority, relating to an alleged lack of safety or regulatory compliance of the Product in the Territory.

1.90 "**Seller**" has the meaning set forth in the preamble.

1.91 "**Seller Account**" has the meaning set forth in Section 5.9.4 (Payments on Account of the Purchased Receivables).

1.92 "**Seller Fundamental Representations**" has the meaning set forth in Section Article 9 (Survival of Representations and Warranties).

1.93 "**Seller Indemnified Party**" has the meaning set forth in Section 7.1 (Indemnification by Purchaser).

1.94 "**Seller IP Assets**" means, individually and collectively, (a) the uniQure Patent Rights (as defined in the License Agreement), (b) the Listed Patent Rights, and (c) any other intellectual property or other proprietary rights of any kind that are owned or held by, or licensed to, Seller.

1.95 "**Seller Percentage Interest**" means, for a given country in the Territory, (a) during the Purchased Receivable Period, 1 minus the ratio of (i) the Purchased Receivables in such Purchased Receivable Period divided by (ii) the Receivables in such Purchased Receivable Period, then multiplied by 100% and (b) during any and all periods prior to or after the Purchased Receivable Period, 100%.

1.96 "**Seller W-8**" has the meaning set forth in Section 6.5 (Tax Forms).

1.97 "**Sublicensee**" means any sublicensee of Licensee under the License Agreement.

1.98 "**Tax**" or "**Taxes**" means any federal, state, local or non-U.S. income, gross receipts, license, payroll, employment, excise, severance, occupation, premium, windfall profits, environmental, customs duties, capital stock, franchise, profits, withholding, social security, unemployment, disability, real property, personal property, abandoned property, value added, alternative or add-on minimum, estimated or other tax of any kind whatsoever, including any interest, penalty or addition thereto, whether disputed or not.

1.99 "**Term**" means the period commencing on the Execution Date and ending on the earlier of (a) the Cap Date, (b) the expiration of the Purchased Receivables Period for all countries in the Territory, or (c) the effective date of termination of the License Agreement.

1.100 "**Termination Date**" has the meaning set forth in Section 8.1 (Termination Date).

1.101 "**Territory**" means worldwide.

1.102 "**Third Party**" means any Person other than Seller or Purchaser or their respective Affiliates.

1.103 "**Total Net Amount**" means, as of any date, (a) the aggregate amount of all payments remitted to, or otherwise received by, Purchaser on or prior to such date pursuant to the Transaction Documents, including (i) all payments in respect of Purchased Receivables pursuant to Section 5.9 (Reports; Payment on Account of the Purchased Receivables) (or otherwise), (ii) the aggregate amount of Proceeds that are remitted to, or otherwise received by, Purchaser pursuant to Section 5.10.3 (License Agreement) or Section 5.11.4 (Listed Patent Rights), and (iii) if applicable, the Rebate Payment made by Seller pursuant to Section 2.6 (Purchase Rebate), *less* (b) (i) all overpayments of Royalty Payments in a country in the Territory required to be, and actually, reimbursed to Licensee pursuant to Section 5.2 (Inspections and Audits of Seller) (or otherwise) on or prior to such date (but only to the extent that such overpayments have been included in the calculation of the Total Net Amount under the immediately preceding clause (a)), and (ii) the aggregate amount of all costs and expenses actually paid by Purchaser (and not actually reimbursed to Purchaser, whether by Seller or any other Person) on or prior to such date pursuant to Section 5.10.3 (License Agreement); provided that any amounts withheld by or on behalf of Seller (or paid directly by Purchaser if asserted, assessed, or otherwise claimed by a Tax authority as a result of a failure to make such withholding) in respect of Indemnified Taxes shall not be included in the Total Net Amount.

1.104 "**Transaction Documents**" means this Agreement and the Bill of Sale.

1.105 "**U.S. Person**" means any "United States person" within the meaning of Section 7701(a)(30) of the Code.

1.106 "**UCC**" means the Uniform Commercial Code (or any similar or equivalent legislation) as in effect in any applicable jurisdiction.

1.107 "**Update Report**" has the meaning set forth in Section 5.1 (Information Rights).

1.108 "**Withheld Party**" has the meaning set forth in Section 5.13.1 (Tax Matters).

1.109 "**Withholding Party**" has the meaning set forth in Section 5.13.1 (Tax Matters).

ARTICLE 2
PURCHASE AND SALE OF THE PURCHASED RECEIVABLES

2.1. **Purchase and Sale**.

2.1.1. **Transfer of Purchased Receivables**. Subject to the terms and conditions of this Agreement, at the Closing, Seller will sell, transfer, assign, and convey to Purchaser, and Purchaser will purchase, acquire, and accept from Seller, Seller's rights, title, and interests in and to the Purchased Receivables, free and clear of any and all Liens.

2.1.2. **Financing Statements**. It is the intention of the Parties that the sale, transfer, assignment, and conveyance contemplated by this Agreement be, and is, a true, complete, absolute, and irrevocable sale, transfer, assignment, and conveyance by Seller to Purchaser of all of Seller's rights, title, and interests in and to the Purchased Receivables. The Purchaser intends to treat the purchase of the Purchased Receivables as a true sale on its books. Neither Seller nor Purchaser intends the transactions contemplated by this Agreement to be characterized or treated as (other than for financial reporting and accounting purposes) a loan from Purchaser to Seller or a financing transaction or a borrowing. It is the intention of the Parties that the beneficial interest in and title to the Purchased Receivables and any "proceeds" (as such term is defined in the UCC) thereof will not be part of Seller's estate in the event of the filing of a petition by or against Seller under any Bankruptcy Laws. Each of Seller and Purchaser hereby waives, to the maximum extent permitted by Applicable Law, any right to contest or otherwise assert that this Agreement does not constitute a true, complete, absolute, and irrevocable sale, transfer, assignment, and conveyance by Seller to Purchaser of all of Seller's rights, title, and interests in and to the Purchased Receivables under Applicable Law, which waiver will, to the maximum extent permitted by Applicable Law, be enforceable against Seller in any Insolvency Event relating to Seller. Accordingly, Seller will treat the sale, transfer, assignment, and conveyance of the Purchased Receivables as a sale of an "account" or a "payment intangible" (as appropriate) in accordance with the UCC, and Seller hereby authorizes Purchaser to file UCC financing statements and any comparable filings in the Netherlands (if applicable) (and continuation statements with respect to such financing statements when applicable) (the "**Financing Statements**") naming Seller as the debtor and Purchaser as the secured party in respect to the Purchased Receivables. Not in derogation of the foregoing statement of the intent of the Parties in this regard, and for the purposes of providing additional assurance to Purchaser in the event that, despite the intent of the Parties, the sale, transfer, assignment, and conveyance contemplated hereby is hereafter held not to be a sale, Seller does hereby grant to Purchaser, as security for the payment of the Purchased Receivables due to

Purchaser as provided herein, a security interest in and to all rights, title, and interests in, to, and under the Purchased Receivables, and Seller does hereby authorize Purchaser, from and after the Closing, to file such Financing Statements (and continuation statements with respect to such Financing Statements when applicable) in such manner and such jurisdictions as are necessary or appropriate to perfect such security interest (the "**Back-Up Security Interest**").

2.2. **Purchase Price**. In full consideration for the sale, transfer, assignment, and conveyance of the Purchased Receivables, and subject to the terms and conditions set forth herein, Purchaser will pay (or cause to be paid) to Seller, or Seller's designee, the following amounts:

2.2.1. $375,000,000, in immediately available funds by wire transfer to the Seller Account on the Closing Date, without any deduction for any withholding or other Taxes, except as required by Applicable Law and in accordance with Section 6.2.1 (Payment of Purchase Price) (the "**Closing Price**"); provided that, so long as Seller's representation in Section 3.19 remains true as of the Closing Date and the Seller has provided the Seller W-8 to Purchaser prior to Closing, Purchaser shall not withhold or deduct any U.S. federal withholding tax unless required to do so as the result of a change in Applicable Law between the date hereof and the Closing Date; and provided, further, that, while Purchaser is not currently aware of any non-U.S. tax required to be deducted or withheld from the payment of the Closing Price to the Seller hereunder, Seller acknowledges and agrees that if Purchaser subsequently becomes aware of any non-U.S. withholding obligation prior to Closing, then Purchaser shall be entitled to withhold and shall not be obligated to pay any additional amounts to Seller in respect of such withholding; provided that, if Purchaser fails to provide Seller with notice of such non-U.S. withholding at least five Business Days prior to Closing (as is required by Section 5.13.1), then Seller shall have the right, but not the obligation, to delay Closing until the sixth Business Day after such notice is given; and

2.2.2. A one-time milestone payment in the event that the aggregate Net Sales of the Product in the Territory during the 12 month period ending December 31, 2024 exceed $[***] (the "**Milestone Event**"), in the amount of $25,000,000 payable in cash no later than 15 Business Days after Purchaser's receipt of a Purchased Receivable report indicating the achievement of the Milestone Event, or at the sole election of Purchaser, Purchaser shall instruct the Depositary Bank to deduct this amount against each subsequent quarterly Purchased Receivable Payment due to Purchaser and pay such amount to Seller no later than 12 Business Days after the Depositary Bank's receipt of such Purchased Receivable Payment(s) (or upon Purchaser's receipt of Purchased Receivables from the Lockbox Account, if earlier), until such milestone payment has been fully applied (the "**Milestone Payment**" and to the extent the Milestone Payment is actually paid to Seller, and together with the Closing Price, the "**Purchase Price**").

2.3. **No Assumed Obligations**. Notwithstanding any provision to the contrary in this Agreement, or any other writing, Purchaser is purchasing, acquiring, and accepting only

the Purchased Receivables and is not assuming any liability or obligation of Seller or any of Seller's Affiliates of whatever nature, whether presently in existence or arising or asserted hereafter, under the License Agreement. All such liabilities and obligations of Seller or Seller's Affiliates will be retained by and remain liabilities and obligations of Seller or Seller's Affiliates, as the case may be (the "**Excluded Liabilities and Obligations**").

2.4. **Excluded Assets**. Purchaser does not, by purchase, acquisition, or acceptance of the rights, title, or interests granted hereunder to the Purchased Receivables, purchase, acquire, or accept any rights, title, or interests in or to any Excluded Assets. Notwithstanding any provision to the contrary set forth in this Agreement, Seller will retain all of its rights, title, and interests in and to all Excluded Assets, and all Excluded Assets will be excluded from the sale, transfer, assignment, and conveyance to Purchaser under this Agreement.

2.5. **Cap Payment Option**. Seller will have the option to make a one-time payment to Purchaser in the amount of $[***] (the "**Cap Payment**"), which Cap Payment will reduce the Cap Amount on a dollar-for-dollar basis, effective as of the date of the Cap Payment, for purposes of determining the Cap Date. Seller may make the Cap Payment to Purchaser any time during the Term.

2.6. **Purchaser Rebate**. In the event that the aggregate Net Sales of the Product in the Territory during the 12 month period ending December 31, 2024 do not equal at least $[***], Seller will pay (or cause to be paid) to the Lockbox Account for the benefit of Purchaser, the amount of $25,000,000, in cash, payable no later than three (3) Business Days following receipt by Purchaser of the first Sales Milestone Payment (as defined in the License Agreement) pursuant to Section 8.2.2 of the License Agreement after December 31, 2024 (the "**Rebate Payment**").

2.7. **No Credits or Refunds**. The Closing Price, the Milestone Payment, and the Rebate Payment, if applicable, will be non-creditable and non-refundable, and except as required by Applicable Law, such payments will not be subject to any withholding or offset or reduction for any Tax; provided that the tax certifications described in Section 6.5 remain valid.

2.8. **Expenses**. Purchaser shall provide to Seller, at least two (2) Business Days prior to the Closing, reasonable documentation with respect to all Third Party expenses incurred by Purchaser and its Affiliates in connection with the negotiation of, and entry into, this Agreement, and the consummation of the transactions contemplated hereby (the "**Purchaser Expenses**").

2.9. **Lockbox Account**.

2.9.1. **Instruction Letter**. Seller will, on or prior to [***] (or, if a deposit account with protections for Purchaser similar to those afforded by a "deposit account" (as defined in Article 9 of the UCC) can not be opened in the Netherlands and Seller must open a deposit account in the U.S., then as promptly as reasonably practicable following such determination), enter into a Deposit Account Control Agreement

with the Depositary Bank with respect to the Lockbox Account. Seller will deliver instructions to Licensee (the "**Instruction to Payor**") with respect to any Purchased Receivables (which instruction will be in form and substance reasonably satisfactory to Purchaser) to remit such Purchased Receivables to the Lockbox Account; provided that if Seller has not established a Depository Account in the Netherlands on or prior to [***], then, notwithstanding any provision to the contrary set forth in this Agreement, (a) Seller will prepay [***]% of the royalty payments due to Purchaser as Purchased Receivables as set forth in the Flash Report (as defined in the License Agreement, with such [***]% premium known as the "**Premium**") no later than [***], and in such case, Seller will immediately commence establishing a deposit account in the U.S. pursuant to a Deposit Account Control Agreement, and (b) Purchaser will direct the Depositary Bank to pay any and all such Premiums of such Purchased Receivables (including, in the event of any dispute with respect thereto, any undisputed portions thereof) to Seller no later than 12 Business Days following the establishment of such Deposit Account Control Agreement. To the extent any Purchased Receivables are paid directly to Seller, Seller will remit to Purchaser all such amounts within 15 Business Days after Seller becoming aware of its receipt of any such funds.

2.9.2. **Transfer of Funds**. Purchaser will have the right to exercise all of its rights and remedies under this Agreement with respect to the Purchased Receivables deposited to the Lockbox Account, including directing the Depositary Bank to transfer all of the funds in respect of the Purchased Receivables in the Lockbox Account to Purchaser. To the extent any Excluded Assets are paid to the Lockbox Account, Purchaser will, absent any dispute between the Parties with respect thereto, direct the Depositary Bank to pay such amounts (including, in the event of any dispute with respect thereto, any undisputed portions thereof) to Seller no later than 12 Business Days following the Depositary Bank's receipt thereof.

2.9.3. **Replacement Lockbox Account**. Seller will have no right to terminate the Lockbox Account or to establish a replacement Lockbox Account with a replacement Depositary Bank without Purchaser's prior written consent. For purposes of this Agreement, any reference to the "Lockbox Account", "Depositary Bank," and "Deposit Account Control Agreement" will refer to such replacement Lockbox Account, Depositary Bank, or Deposit Account Control Agreement, as the context requires.

ARTICLE 3
REPRESENTATIONS OF SELLER

Except as set forth on Exhibit C, Seller hereby represents to Purchaser as of the Execution Date as follows:

3.1. **Existence**. Seller is a private company duly organized, validly existing, and in good standing under the laws of the Netherlands. Seller has all legal powers and all licenses, authorizations, consents, and approvals required to do business in each jurisdiction in which the nature of the business conducted by it or the character or location of the

properties and assets owned, leased, or operated by it makes such licenses, authorizations, consents, and approvals necessary, except where the failure to be so licensed, authorized, consented, and approved has not and would not reasonably be expected to have, either individually or in the aggregate, a Material Adverse Effect.

3.2. **Authorization**. Seller has all requisite corporate power and authority to execute, deliver, and perform its obligations under this Agreement. The execution, delivery, and performance of this Agreement, and the consummation of the transactions contemplated hereby, have been duly authorized by all necessary corporate action on the part of Seller.

3.3. **Enforceability**. This Agreement has been duly executed and delivered by an authorized officer of Seller and constitutes the valid and binding obligation of Seller, enforceable against Seller in accordance with its terms, except as may be limited by applicable Bankruptcy Laws or by general principles of equity (whether considered in a proceeding in equity or at law).

3.4. **No Conflicts**. The execution, delivery, and performance by Seller of this Agreement and the consummation of the transactions contemplated hereby do not and will not (a) contravene or conflict with the articles of association of Seller, (b) contravene or conflict with or constitute a material default under any law binding upon or applicable to Seller, or (c) contravene or conflict with or constitute a material default under any material agreement or Judgment binding upon or applicable to Seller.

3.5. **Consents**. Except for the consents that will have been obtained on or prior to the Closing, the Financing Statements contemplated by Section 2.1.2 (Financing Statements), the Deposit Account Control Agreement with the Depositary Bank with respect to the Lockbox Account, or any filings required by the federal securities laws or stock exchange rules, no consent, approval, license, order, authorization, registration, declaration, notice, or filing with or of any Governmental Authority or other Person is required to be done or obtained by Seller in connection with (a) the execution and delivery by Seller of this Agreement, (b) the performance by Seller of its obligations under this Agreement (including delivery of any Reports to Purchaser and its Affiliates and their respective Representatives), or (c) the consummation by Seller of any of the transactions contemplated by this Agreement.

3.6. **Compliance**.

3.6.1. All applications, submissions, information, and data related to the Product in the Territory submitted or utilized as the basis for any request to any Regulatory Authority by or on behalf of Seller, were true and correct in all material respects as of the date of such submission or request, and any material updates, changes, corrections, or modification to such applications, submissions, information, or data required under Applicable Laws or regulations to be made by or on behalf of Seller have been submitted to the necessary Regulatory Authorities.

3.6.2. Neither Seller nor any of its Affiliates 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 or EMA to invoke its policy with respect to “Fraud, Untrue

Statements of Material Facts, Bribery, and Illegal Gratuities", or similar policies, set forth in any Applicable Laws or regulations.

3.6.3. Seller has provided to Purchaser, at least five (5) days prior to the date hereof, in a data room available to Purchaser true and correct copies or summaries of all material written communications sent or received by Seller and any of its Affiliates to or from any Regulatory Authorities in the Territory, as applicable, that relate to the Product since [***], provided that, with respect to any request of Purchaser made within ten (10) days prior to the date hereof, Seller has provided such copies or summaries to Purchaser at least one (1) Business Day prior to the date hereof.

3.6.4. None of Seller, any of its Affiliates and, to the Knowledge of Seller, any Third Party manufacturer of any Product, has received from the FDA a "Warning Letter", Form FDA-483, "Untitled Letter," or similar material correspondence or notice alleging violations of Applicable Laws and regulations enforced by the FDA, or any comparable correspondence from any other Regulatory Authority in the Territory, as applicable, with regard to the Product or the manufacture, processing, packaging, or holding thereof, the subject of which communication is unresolved and if determined adversely to Seller or such Affiliate would, individually or in the aggregate, reasonably be expected to result in a Material Adverse Effect.

3.6.5. Since [***], (a) there have been no Safety Notices in the Territory, as applicable, (b) there are no unresolved material Product complaints with respect to the Products, and (c) to the Knowledge of Seller, there are no facts currently in existence that would, individually or in the aggregate, reasonably be expected to result in (i) a material Safety Notice with respect to the Product, or (ii) a material change in the labeling of the Product. Since [***], neither Seller nor any of its Affiliates has experienced any significant failures in the manufacturing of the Product for clinical use or commercial sale in the Territory, as applicable that, individually or in the aggregate, have had or would reasonably be expected to result in, if such failure occurred again, a material and adverse impact on the Purchased Receivables.

3.7. **No Liens; Title to Purchased Receivables**. None of the Product Rights owned by Seller or its Affiliates is subject to any Liens other than Permitted Liens. None of the material property or assets owned by Seller or its Affiliates, in each case, to the extent specifically related to the Products in the Territory, as applicable, is subject to any Lien, except for Permitted Liens. Seller has good and marketable title to the Purchased Receivables, free and clear of all Liens, except for any Permitted Liens. Upon the Closing, Purchaser will have acquired, subject to the terms and conditions set forth in this Agreement, good and marketable title to the Purchased Receivables, free and clear of all Liens.

3.8. **Lien-Related Representation and Warranties**. Seller's exact legal name is, and for the prior five years has been, "uniQure biopharma BV". Seller is a private company duly organized, validly existing, and in good standing under the laws of the Netherlands, located at Paasheuvelweg 25a, 1105 BP Amsterdam, the Netherlands.

3.9. **Subordination**. Seller has not caused, by any means, the Purchased Receivables to be subordinated to the rights of any creditor of Licensee or any other Person. In addition, Seller has not caused and, to the Knowledge of Seller, no other Person has caused, the claims and rights of Purchaser created by this Agreement in and to the Purchased Receivables to be subordinated to any creditor of Licensee or any other Person; provided, *however*, that Seller makes no representation as to whether Purchaser (or any Person acting on behalf of Purchaser) has caused any such subordination.

3.10. **No Litigation**. There is no pending, or to the Knowledge of Seller, threatened, action, suit, proceeding, or investigation before any Governmental Authority, court, or arbitrator against Seller that, individually or in the aggregate, (a) would reasonably be expected to result in a Material Adverse Effect or (b) challenges or seeks to prevent or delay the consummation of any of the transactions contemplated by the Transaction Documents.

3.11. **Brokers' Fees**. There is no investment banker, broker, finder, financial advisor, or other intermediary who has been retained by or is authorized to act on behalf of Seller who might be entitled to any fee or commission in connection with the transactions contemplated by this Agreement other than Moelis & Company (whose fees and commissions are solely the responsibility of Seller).

3.12. **Compliance with Laws**. Seller is not in violation of and, to the Knowledge of Seller, is not under investigation with respect to and has not been threatened to be charged with any material violation of, any Applicable Law or any Judgment, order, injunction, or decree of any Governmental Authority relating to the Product or the License Agreement.

3.13. **Insolvency Event**. No Insolvency Event exists regarding Seller or any of its Affiliates.

3.14. **Material Adverse Effect**. To the Knowledge of Seller, (a) except as disclosed to Seller in writing prior to the Execution Date, no event has occurred and no condition exists that would reasonably be expected to result in an adverse effect on the timing, duration, or amount of the Purchased Receivables and (b) no event has occurred and no condition exists that would reasonably be expected to result in any one or more of: (i) a material adverse effect on the right or ability of Seller to consummate the transactions contemplated by the Transaction Documents and perform its obligations under the Transaction Documents, (ii) a material adverse effect on the validity or enforceability of the Transaction Documents against Seller or the rights of Purchaser thereunder, or (iii) a material adverse effect on the rights of Seller under the License Agreement.

3.15. **Intellectual Property Matters**.

3.15.1. The Listed Patent Rights, as set forth on Exhibit B, constitute all of the uniQure Royalty Patent Rights (as defined in the License Agreement), except as otherwise disclosed on Section 3.15.1 of Exhibit C.

3.15.2. For each of the Listed Patent Rights, Seller has indicated on Exhibit B (a) the jurisdictions in which such Patent Right is pending, allowed, granted, or issued, (b) the patent number or patent serial number, and (c) the scheduled initial expiration

date, including a notation if such scheduled expiration date includes a term extension. Seller has clear title to the Listed Patent Rights.

3.15.3. Except as set forth on Section 3.15.3 of Exhibit C, the Listed Patent Rights that have been issued or granted by the appropriate Patent Office are valid and, to the Knowledge of Seller, enforceable. Seller has not, and to the Knowledge of Seller, Licensee has not, received (a) any opinion of counsel (other than any unsolicited opinion of counsel of any Third Party) that any of the Listed Patent Rights is invalid or unenforceable, or (b) any notice of any claim by any Third Party challenging the validity or enforceability of any of the Listed Patent Rights.

3.15.4. Except as set forth on Section 3.15.4 of Exhibit C, within each family of related patent filings within the Listed Patent Rights, (a) in each jurisdiction in which a patent has issued, there is at least one valid claim that would be infringed by Licensee's manufacture, use, import, offering for sale, or sale of the Products, but for Licensee's rights in the Listed Patent Rights, and (b) in each jurisdiction in which a patent application has been filed but not yet issued, to the knowledge of the Chief Executive Officer, Chief Financial Officer, Chief Legal Officer, Vice President, Intellectual Property, and President, Research & Development of Seller, following reasonable inquiry, (i) there is one or more pending claims that, if issued as filed, would cover the manufacture, use, import, offering for sale, or sale of the Licensed Product and (ii) except as available in the prosecution history of the applicable pending claim, there are no facts or circumstances precluding such pending claims from issuing in due course.

3.15.5. To the Knowledge of Seller, the Listed Patent Rights have been prosecuted in accordance with Applicable Law. To the Knowledge of Seller, each individual involved in the filing and prosecution of the Listed Patent Rights has complied in all material respects with all applicable duties of candor and good faith in dealing with the United States Patent Office in connection with the filing and prosecution of the Listed Patent Rights.

3.15.6. To the Knowledge of Seller, (a) there are no unpaid maintenance fees payable by Seller to any Governmental Authority that currently are overdue for any of the Listed Patent Rights and (b) no Listed Patent Rights have lapsed or been abandoned, cancelled, or expired.

3.15.7. There is no pending or, to the Knowledge of Seller, threatened opposition, interference, reexamination, injunction, claim, suit, action, citation, summon, subpoena, hearing, inquiry, investigation (by the International Trade Commission or otherwise), complaint, arbitration, mediation, demand, decree, or other dispute, disagreement, proceeding, or claim (collectively, "**Disputes**") challenging the legality, validity, scope, enforceability, or ownership of any of the Listed Patent Rights and that would give rise to any Royalty Deduction against the payments due to Seller under the License Agreement. To the Knowledge of Seller, there are no pending or threatened Disputes by Licensee, or its Affiliates or sublicensees, challenging the legality, validity, scope, enforceability, or ownership of any of the

Listed Patent Rights or that would give rise to any Royalty Deduction against the payments due to Seller under the License Agreement. The Listed Patent Rights are not subject to any outstanding injunction, Judgment, order, decree, ruling, change, settlement, or other disposition of a Dispute. There are no proceedings, other than proceedings in the ordinary course of patent prosecution and except as set forth in Schedule 3.15.7, with respect to the Listed Patent Rights listed on Exhibit B.

3.15.8. Except as set forth on Section 3.15.8 of Exhibit C, there is no pending action, suit, proceeding, investigation, or claim and, to the Knowledge of Seller, there is no threatened action, suit, proceeding, investigation, or claim, and, to the Knowledge of Seller, no event has occurred or circumstance exists that (with or without notice or lapse of time, or both) would reasonably be expected to give rise to or serve as a basis for any action, suit, proceeding, investigation, or claim by any Person that claims that the manufacture, use, marketing, sale, offer for sale, importation, or distribution of the Product does or could infringe on any patent or other intellectual property rights of any Third Party or constitute misappropriation of any other Person's trade secrets or other intellectual property rights. To the Knowledge of Seller, there are no patents issued, and no pending patent applications, owned by any Third Party that, if issued, would limit or prohibit, in any material respect, the manufacture, use, or sale of the Product by Seller, Licensee, or any of its respective sublicensees.

3.15.9. To the knowledge of the Chief Executive Officer, Chief Financial Officer, Chief Legal Officer, Vice President, Intellectual Property, and President, Research & Development of Seller, following reasonable inquiry and except as set forth in Section 3.15.9 of Exhibit C, no patent or other intellectual property rights of any other Person has been, or is, or will be, infringed by Licensee, Seller or Purchaser's Exploitation of the Products. Seller has not received, and to the Knowledge of Seller, Licensee has not received, any opinion of counsel regarding infringement or non-infringement of any patent or other intellectual property rights of any other Person by Licensee's Exploitation of any Product.

3.15.10. Seller and, to the Knowledge of Seller, Licensee has taken all reasonable precautions to protect the secrecy and confidentiality of the uniQure Know-How (as defined in the License Agreement).

3.15.11. There is no Person who is or claims to be an inventor under any Listed Patent Right who is not a named inventor thereof. The list of inventors named in each issued and unexpired Listed Patent Right listed on Exhibit B is current and complete in accordance with the laws of the jurisdiction in which such Listed Patent Right was issued.

3.15.12. Except as set forth on Section 3.15.12 of Exhibit C, (a) the Listed Patent Rights are not subject to any encumbrance, lien, or claim of ownership by any Third Party, and there are no facts that would preclude Seller from having unencumbered title to the Listed Patent Rights, and (b) Seller has not received any notice of any

claim by any Third Party challenging the ownership of the rights of Seller in and to the Listed Patent Rights.

3.15.13. Each Person who has or has had any rights in or to the Listed Patent Rights, including each inventor named on such Listed Patent Rights, has executed a contract, or is otherwise subject to a written instrument, assigning their entire right, title and interest in and to such Listed Patent Rights and the inventions embodied, described or claimed therein, to the owner thereof. With respect to the United States Listed Patent Rights, each such Listed Patent Right includes a duly recorded assignment at the United States Patent and Trademark Office.

3.15.14. Except as set forth on Section 3.15.14 of Exhibit C, on a country by country basis, in at least the United States and E.U., (A) (i)Licensee has not exercised any step-down pursuant Section 8.3.2(a) of the License Agreement, and, (ii) to the Knowledge of Seller, no event or condition exists that, upon notice or passage of time, or both, would permit Licensee to exercise any step-down pursuant to Section 8.3.2(a) of the License Agreement during the period in which AMT-061 is protected by regulatory exclusivity in the United States and applicable E.U. country; and (B) (i) Licensee has not exercised any step-down pursuant Section 8.3.2(b) of the License Agreement, and (ii) no step-down pursuant to Section 8.3.2(b) of the License Agreement shall occur during the period in which AMT-061 is protected by regulatory exclusivity in the United States and applicable E.U. country.

3.15.15. Seller has not received from Licensee, any notice of termination of the License Agreement or any indication, whether written or oral, of a suggestion or intent to terminate the License Agreement.

3.16. **License Agreement.**

3.16.1. **Copy**. Schedule 3.16.1 hereto contains a true and complete copy of the License Agreement (including all amendments, supplements, and other modifications or restatements thereto) as of the Execution Date.

3.16.2. **No Unpaid Royalty Payments**. To the Knowledge of Seller, other than the Royalty Payment for the calendar quarter (or portion thereof) ended immediately preceding the Execution Date, if any, there are no unpaid Royalty Payments that have become due or are overdue, and none are expected to become overdue as of the Execution Date, in each case, subject to the terms of the License Agreement.

3.16.3. **No Breaches or Defaults**. Seller is not in material breach of the License Agreement. To the Knowledge of Seller, no circumstances or grounds exist that would give rise (a) to a claim by Licensee of a material breach of the License Agreement, or (b) to a right of rescission, termination, revision, setoff, or any other rights, in, to, or under the Purchased Receivables.

3.16.4. **No Termination**. (a) To the Knowledge of Seller, nothing has occurred and no condition exists that would permit either party thereto to terminate the License Agreement for material breach, and (b) Seller has not received any notice of

termination from Licensee under Section 13.2.1 (Termination Without Cause) of the License Agreement. Licensee has not provided Seller with notice of termination of the License Agreement, nor any notice that it plans to terminate the License Agreement.

3.16.5. **Validity; Binding**. The License Agreement and each Existing In-License is valid and binding on each other party thereto in accordance with its terms, subject to bankruptcy, insolvency, reorganization, moratorium, ad hoc representative appointment, conciliation, safeguard proceedings, judicial receivership, or other laws affecting creditors' rights generally or general equitable principles, and is in full force and effect.

3.16.6. **No Sublicenses**. Except as set forth on Schedule 3.16.6, (a) to the Knowledge of Seller, Licensee has not entered into any sublicense under the License Agreement with respect to the Territory, and (b) Seller has not consented to Licensee entering into any sublicense under the License Agreement with respect to the Territory.

3.16.7. **Payments Made**. All payments due and payable by Seller or, to the Knowledge of Seller, the Licensee, under the License Agreement have been timely paid.

3.16.8. **No Indemnification Claims**. There have been no claims for indemnification made under Article 11 (Indemnification) of the License Agreement by any Indemnified Party (as defined in the License Agreement).

3.16.9. **No Offset**. There are no offsets being taken by Licensee pursuant to Section 8.3.2(c) of the License Agreement, and Seller is not aware of any patents or other intellectual property rights of any other Person that form the basis for an offset pursuant to Section 8.3.2(c) of the License Agreement.

3.16.10. **Royalty Payments**. Seller has not:

(a) forgiven, released, delayed, postponed, or compromised any payment in respect of any Royalty Payments;

(b) waived, amended, cancelled or terminated, exercised, or, to the Knowledge of Seller failed to exercise, any material rights constituting or relating to the Purchased Receivables;

(c) except as set forth in Schedule 3.16.10, amended, modified, restated, cancelled, supplemented, terminated, or waived any provision of the License Agreement, or granted any consent thereunder, or agreed to do any of the foregoing;

(d) exercised any right of rescission, offset, counterclaim, or defense, upon or with respect to the Purchased Receivables, or agreed to do or suffer to exist any of the foregoing;

(e) sold, leased, pledged, licensed, transferred, or assigned (or attempted to do any of the foregoing) all or any portion of the Purchased Receivables, except in favor of Purchaser pursuant to this Agreement; or

(f) received any advance payments on any Royalty Payments.

3.16.11. **Other Licenses**. Except for the Existing In-Licenses and as set forth on Schedule 3.16.11, the License Agreement constitutes the only agreement (a) to which Seller is a party relating to the Royalty Payments, (b) that relates to Seller's entitlement to the Purchased Receivables, or (c) that relates to the Development (as defined in the License Agreement) or Commercialization (as defined in the License Agreement) of the Product in the Territory.

3.17. **Disclosure**. Seller has made available to Purchaser all the information that Purchaser has requested for deciding whether to acquire the Purchased Receivables. No representation or warranty of Seller contained in this Agreement, as qualified by Exhibit C, and no certificate furnished or to be furnished to Purchaser at the Closing contains any untrue statement of a material fact or, to the Knowledge of Seller, omits to state a material fact necessary in order to make the statements contained herein or therein not materially misleading in light of the circumstances under which they were made.

3.18. **Taxes**. No deduction or withholding for or on account of any Tax has been made from any payment by Licensee to Seller under the License Agreement, and, except with respect to U.S. federal withholding tax, Licensee has not requested Seller to establish any entitlement to any treaty benefits in order to avoid any such withholding.

3.19. **Withholding**. Seller is, and on the Closing Date will be, exempt from U.S. federal withholding Tax on all payments made by Purchaser with respect to the Purchased Receivables by reason of Seller being a qualified resident of the Netherlands for purposes of the U.S./Netherlands tax treaty that is entitled to a zero rate of U.S. federal withholding on both royalties, other income, and interest.

ARTICLE 4
REPRESENTATIONS AND WARRANTIES OF PURCHASER

Purchaser hereby represents and warrants to Seller as of the Execution Date as follows:

4.1. **Existence; Good Standing**. Purchaser is an entity duly organized, validly existing, and in good standing under the laws of the respective jurisdiction in which it is organized.

4.2. **Authorization**. Purchaser has the requisite limited partnership right, power, and authority to execute, deliver, and perform its obligations under this Agreement. The execution, delivery, and performance of this Agreement, and the consummation of the transactions contemplated hereby, have been duly authorized by all necessary action on the part of Purchaser.

4.3. **Enforceability**. This Agreement has been duly executed and delivered by an authorized Person of Purchaser and constitutes the valid and binding obligation of Purchaser,

enforceable against Purchaser in accordance with its terms, except as may be limited by applicable Bankruptcy Laws or by general principles of equity (whether considered in a proceeding in equity or at law).

4.4. **No Conflicts**. The execution, delivery, and performance by Purchaser of this Agreement do not and will not (a) contravene or conflict with the organizational documents of Purchaser, (b) contravene or conflict with or constitute a default under any material provision of any law binding upon or applicable to Purchaser, or (c) contravene or conflict with or constitute a default under any material contract or other material agreement or Judgment binding upon or applicable to Purchaser.

4.5. **Consents**. Except for any filings required by the federal securities laws or stock exchange rules, no consent, approval, license, order, authorization, registration, declaration or filing with or of any Governmental Authority or other Person is required to be done or obtained by Purchaser in connection with (a) the execution and delivery by Purchaser of this Agreement, (b) the performance by Purchaser of its obligations under this Agreement, or (c) the consummation by Purchaser of any of the transactions contemplated by this Agreement.

4.6. **No Litigation**. There is no action, suit, investigation, or proceeding pending or, to the knowledge of Purchaser, threatened before any Governmental Authority to which Purchaser is a party that would, if determined adversely, reasonably be expected to prevent or materially and adversely affect the ability of Purchaser to perform its obligations under this Agreement.

4.7. **Financing**. Purchaser will have sufficient cash to pay the Closing Price at the Closing and when due pursuant to Section 2.2.1 (Purchase Price) and Section 6.2.1 (Payment of Purchase Price). Purchaser acknowledges that its obligations under this Agreement are not contingent on obtaining financing.

4.8. **Brokers' Fees**. There is no investment banker, broker, finder, financial advisor, or other intermediary who has been retained by or is authorized to act on behalf of Purchaser who might be entitled to any fee or commission in connection with the transactions contemplated by this Agreement.

4.9. **Tax Status**. Purchaser is, and on the Closing Date will be, exempt from U.S. federal withholding Tax on all payments made by Seller or Licensee with respect to the Purchased Receivables by reason of Purchaser being a U.S. Person.

ARTICLE 5
COVENANTS

The Parties covenant and agree as follows:

5.1. **Information Rights**. From and after the Closing Date, Representatives of Seller will meet at the request of Purchaser (no more often than once per calendar quarter) to discuss, among other things, material commercial, regulatory and intellectual property developments relating to the Products in the Territory, as applicable, (each such meeting and, if applicable,

related materials provided in response to Purchaser's request for additional information, an "**Update Report**"). Notwithstanding the foregoing limitations, Seller will also provide Purchaser with such additional information in its possession and control regarding the Product in the Territory, as applicable, included in each Update Report as Purchaser may reasonably request from time to time. Seller and its controlled Affiliates will prepare and maintain, and will use reasonable efforts to cause its non-controlled Affiliates and Licensee to prepare and maintain, reasonably complete and accurate records of the information to be disclosed in each Report. All Reports, and the Confidential Information contained therein, will be the Confidential Information of Seller and subject to the obligations of confidentiality set forth in Section 5.8 (Confidentiality).

5.2. **Inspections and Audits of Seller**. Following the Closing, upon at least 30 days' written notice and during normal business hours, no more frequently than once per calendar year, Purchaser may cause an inspection or audit by an independent public accounting firm of recognized international standing ("**Accounting Firm**") to be made of Seller's books of account for the calendar years prior to the audit for the purpose of determining the correctness of Purchased Receivable Payments made under this Agreement. Upon Purchaser's reasonable request, no more frequently than once per calendar year, Seller will use reasonable efforts to exercise any rights it may have under the License Agreement to cause an inspection or audit by an Accounting Firm to be made of the books of account of Licensee for the purpose of determining the correctness of Purchased Receivable Payments made under this Agreement. All of the out-of-pocket expenses of any inspection or audit requested by Purchaser hereunder (including the fees and expenses of such Accounting Firm designated for such purpose) will be borne solely by Purchaser, unless the Accounting Firm determines that Purchased Receivable Payments previously paid during the period of the audit were underpaid by an amount greater than [***]% of the Purchased Receivable Payments actually paid during such period, in which case such expenses will be borne by Seller. Such Accounting Firm will enter into a customary confidentiality agreement and an engagement letter reasonably acceptable to Seller (such consent not to be unreasonably withheld, conditioned, or delayed) governing the use and disclosure of Seller's information disclosed to such Accounting Firm and such Accounting Firm's acceptance of the procedures set forth in this Section 5.2 (Inspections and Audits of Seller). Such Accounting Firm will not disclose the confidential information of Seller or Licensee relating to the Products to Purchaser, except to the extent such disclosure is reasonably necessary to determine the correctness of Purchased Receivable Payments or otherwise would be included in a Report. All information obtained by Purchaser as a result of any such inspection or audit will be Confidential Information of Seller subject to Section 5.8 (Confidentiality). The Parties agree that the calculation of Receivables and the Purchased Receivable Payments by such Accounting Firm contemplated by this Section 5.2 (Inspections and Audits of Seller) is to measure Receivables and the Purchased Receivable Payments in accordance with the terms of this Agreement, and such calculation is not intended to permit the introduction of accounting methods, policies, principles, practices, procedures, classifications, or estimation methodologies contrary to those specified in this Agreement for the purposes of determining Receivables and the Purchased Receivable Payments. Such Accounting Firm will provide a copy of its report to the Parties simultaneously. The Parties will have 30 days from the date of delivery of such report to provide the Accounting Firm with comments on such report, which each Party will deliver

to the Accounting Firm and the other Party simultaneously. The Accounting Firm will consider such comments in good faith and will deliver an updated report within 15 days of the earlier to occur of such 30 day review period or the Parties' written confirmation of submission of final comments to such Accounting Firm's initial report. If the final report of the Accounting Firm in respect of an audit discloses any underpayments by Seller to Purchaser, then such underpayment, will be paid by Seller to Purchaser within 30 days of it being so disclosed. If any audit discloses any overpayments by Seller to Purchaser, then, Seller will have the right to credit the amount of the overpayment against each subsequent quarterly Purchased Receivable Payment due to Purchaser until the overpayment has been fully applied. If the overpayment is not fully applied prior to the final quarterly Purchased Receivable Payment due hereunder, Purchaser will promptly refund an amount equal to any such remaining overpayment.

5.3. **Efforts to Consummate Transactions**. Subject to the terms and conditions of this Agreement, each of Seller and Purchaser will use commercially reasonable efforts prior to the Closing to take, or cause to be taken, all actions and to do, or cause to be done, all things reasonably necessary under Applicable Law to consummate the transactions contemplated by this Agreement. Each of Purchaser and Seller agrees to execute and deliver such other documents, certificates, agreements, and other writings and to take such other actions as may be reasonably necessary in order to consummate or implement expeditiously the transactions contemplated by this Agreement.

5.4. **Further Assurances**. After the Closing, Seller and Purchaser agree to execute and deliver such other documents, certificates, agreements, and other writings and to take such other actions as may be reasonably necessary in order to give effect to the transactions contemplated by this Agreement.

5.5. **Signing Deliveries**. Prior to or simultaneously with the execution of this Agreement, Seller will deliver to Purchaser a certificate of the Secretary or an Assistant Secretary of Seller, dated the date hereof, certifying as to (a) the incumbency of each officer of Seller executing this Agreement and (b) the attached thereto copies of (i) Seller's commercial register excerpt, and (ii) articles of association.

5.6. **Back-Up Security Interest**. Seller and Purchaser agree that notwithstanding any provision to the contrary set forth in this Agreement, except as may be limited by applicable Bankruptcy Laws or by general principles of equity (whether considered in a proceeding in equity or at law), Seller will not enter into any contracts or arrangement or otherwise knowingly take any action or knowingly fail to act in a manner that would, individually or in the aggregate, reasonably be expected to materially and adversely affect Purchaser's ownership of the Purchased Receivables or the Back-Up Security Interest or Purchaser's right to file Financing Statements in accordance with Section 2.1.2 (Financing Statements).

5.7. **Use of Names**. Neither Party, nor any Affiliate or Representative of any Party shall, without the other Party's prior written consent (which will be subject to Section 9.7 (Publicity; Use of Names) of the License Agreement with respect to Licensee and certain of its representatives), identify Purchaser, Seller, Licensee, its or their respective Affiliates, or its or its respective Affiliates' trustees, directors, managers, officers, or employees in

any advertising, press releases, sales literature, or other promotional materials to be disseminated to any Person other than its Affiliates and the employees, officers, directors, investors (and potential investors), agents, and advisors of such Party and its Affiliates except if and to the extent that any such release or disclosure is required by Applicable Law or by any Governmental Authority of competent jurisdiction, including in connection with such Party's filings with the Securities and Exchange Commission, its successor or foreign equivalent, in which case, Seller and Purchaser, or their respective Affiliates, as the case may be, may make such release or disclosure in accordance with the provisions of Section 5.8.2 (Permitted Disclosures) and, with respect to any such press release, shall use commercially reasonable efforts to consult in good faith with the other Party regarding the form and content thereof before issuing such release. Notwithstanding the foregoing, it is the intent of Purchaser and Seller to cooperate with respect to the release of a public disclosure regarding the transactions contemplated by the Transaction Documents on or about the Execution Date and each Party shall be permitted to use the name of the other Party and describe the transactions contemplated by the Transaction Document, to the extent in the public domain, in connection with ordinary course marketing by such Party.

5.8. **Confidentiality**.

5.8.1. **Confidentiality**. Purchaser will keep confidential and not disclose to any Person (other than its Affiliates or any of its or its Affiliates' Representatives), and will cause its Affiliates or any of its or its Affiliates' Representatives to keep confidential and not disclose to any Person, any and all Confidential Information. Purchaser will, and will cause its Affiliates or any of its or its Affiliates' Representatives to, use the Confidential Information solely in connection with Purchaser's administration of the Transaction Documents (and not for any other purpose). The foregoing obligations will continue until the later of (a) (i) the first anniversary of the expiration of the Term or (ii) with respect to any Confidential Information disclosed by Seller to Purchaser and clearly and conspicuously identified as subject to the confidentiality obligations under the License Agreement, the 10th anniversary of the expiration of the Term and (b) the date of expiration of the last to expire of the obligations of Purchaser under Section 5.8.5.

5.8.2. **Permitted Disclosures**. If Purchaser or its Affiliates or any of its or its Affiliates' Representatives are requested or required by Applicable Law or the regulations of a stock exchange or Governmental Authority or Regulatory Authority or by the order or ruling of a court, administrative agency, or other government body of competent jurisdiction to disclose any Confidential Information, then Purchaser will promptly, and, in any event, use reasonable efforts to promptly upon learning of such request or requirement, to the extent permitted by Applicable Law, notify Seller in writing of such request or requirement so that Seller may seek an appropriate protective order or other appropriate remedy (and if Seller seeks such an order or other remedy, Purchaser will provide such cooperation, at Seller's expense, as Seller will reasonably request). If no such protective order or other remedy is obtained and Purchaser or its Affiliates or its or its Affiliates' Representatives are, in the view of their respective counsel (which may include their respective internal counsel), legally compelled to disclose Confidential

Information, Purchaser or its Affiliates or its or its Affiliates' Representatives, as the case may be, will only disclose that portion of the Confidential Information that their respective counsel advises that Purchaser or its Affiliates or its or its Affiliates' Representatives, as the case may be, are compelled to disclose and will exercise reasonable efforts, at Seller's expense, to obtain reliable assurance that confidential treatment will be accorded to that portion of the Confidential Information that is being disclosed. In any event, Purchaser will not oppose action by Seller to obtain an appropriate protective order or other reliable assurance that confidential treatment will be accorded the Confidential Information.

5.8.3. **Termination of Confidentiality Agreement**. Effective upon the Execution Date, the Confidentiality Agreement will terminate and be of no further force or effect, and will be superseded by the provisions of this Section 5.8 (Confidentiality).

5.8.4. **Specific Enforcement**. Purchaser stipulates and agrees that remedies at law may not be adequate to protect Seller against any actual or threatened breach of this 5.8 (Confidentiality) by Purchaser, its Affiliates, or its or its Affiliates' Representatives, and that Seller will be entitled to seek specific performance and temporary and permanent injunctive relief or other equitable relief as a remedy for any such actual or threatened breach.

5.8.5. **Other Relevant Obligations**. In addition to, and without limiting, Purchaser's obligations under this Section 5.8 (Confidentiality), Purchaser will fully comply with any confidentiality obligations of Seller or any of its Affiliates under the License Agreement that are applicable to the Confidential Information arising therefrom.

5.9. **Reports; Payments on Account of the Purchased Receivables**.

5.9.1. From and after the Closing Date, during the Purchased Receivable Period, for each calendar quarter, promptly, but in any event no later than 15 days after receipt of the Royalty Report from Licensee for such calendar quarter, Seller will provide to Purchaser a report setting forth in reasonable detail (a "**Purchased Receivable Report**"): (a) the Receivables for the applicable calendar quarter, on a country-by-country and Product-by-Product basis, and (b) (i) the calculation of the Purchased Receivable Payment payable to Purchaser for the applicable calendar quarter; provided that Purchased Receivable Payments based upon Receivables will be included in a Purchased Receivable Report to the extent such Receivable is actually received by Seller in the applicable calendar quarter; and (ii) all Existing In-License Payments due in such calendar quarter.

5.9.2. Seller will make all payments required to be made by it to Purchaser pursuant to this Agreement by wire transfer of immediately available funds to the account(s) set forth in the Payment Direction and Confidentiality Letter (or to such other account as Purchaser will notify Seller in writing from time to time) (the "**Purchaser Account**"):

5.9.3. If Purchaser receives any future payment from Licensee, any Sublicensee, or any other Person (including Seller) that constitutes (a) an Excluded Asset, (b) a Royalty Payment that does not consist entirely of Purchased Receivables, or (c) a Total Net Amount that exceeds the Cap Amount, then (i) such payment will be held by Purchaser in trust for the benefit of Seller, (ii) Purchaser will have no rights, title, or interests whatsoever in such payment, and (iii) Purchaser promptly, and in any event no later than 10 Business Days following Purchaser becoming aware of its receipt of such payment, will remit such payment to the Seller Account pursuant to Section 5.9 (Reports; Payments on Account of the Purchased Receivables).

5.9.4. Purchaser will make all payments required to be made by it to Seller pursuant to this Agreement by wire transfer of immediately available funds to the account(s) set forth in the Payment Direction and Confidentiality Letter (or to such other account as Seller will notify Purchaser in writing from time to time) (the "**Seller Account**"):

5.9.5. Notwithstanding any provision to the contrary set forth in this Agreement, Seller will have no obligation to make any payment with respect to Purchased Receivables to Purchaser until Seller will have actually received such Purchased Receivables from Licensee.

5.10. **License Agreement**.

5.10.1. During the Purchased Receivable Period, Seller (a) will not breach any of the provisions of the License Agreement in a manner that would adversely affect in any respect the amount, timing, duration, or value of the Purchased Receivables, (b) will not forgive, release, or compromise any amount owed to or becoming owed to it under the License Agreement, which amount constitutes Purchased Receivables hereunder, without Purchaser's prior written consent, and (c) will not assign, amend, modify, supplement, restate, waive, cancel, or terminate (or consent to any cancellation or termination of), in whole or in part, any rights affecting the Purchased Receivables in a manner that would reasonably be expected to adversely affect the value of the Purchased Receivables, without Purchaser's prior written consent. Notwithstanding the foregoing, Purchaser stipulates and agrees that it will not be a breach of this Agreement, and no consent of Purchaser will be required, for Seller to allow, without interest or penalty, Licensee to remit to Seller Royalty Reports, Purchased Receivable Reports and Royalty Payments on behalf of Seller in accordance with the terms of this Agreement.

5.10.2. During the Purchased Receivable Period, promptly after a Responsible Seller Party obtains Knowledge of a breach of or default under, or an alleged breach of or default under the License Agreement by Licensee that would reasonably be expected to adversely affect in any material respect the value of the Purchased Receivables, Seller will (a) promptly (but in any event within five Business Days) give a written notice to Purchaser describing in reasonable detail (to the extent such description would not constitute a Confidentiality Breach) the relevant breach or default and (b) proceed in consultation with Purchaser (and the Royalty Parties, as applicable).

Seller will enforce compliance within the time periods set forth in Section 8.3.3 (Royalty Payments and Reports) of the License Agreement (or to give written notice of any such noncompliance to Purchaser), and seek payment of interest under Section 8.11 (Late Payments) of the License Agreement, unless Purchaser consents in writing to waive or defer such obligation.

5.10.3. Any Proceeds of an enforcement of Licensee's obligations under the License Agreement pursuant to this Section 5.10 (License Agreement), after deduction of all costs and expenses (including attorneys' fees and expenses) incurred by [***] in connection with such enforcement, will be allocated between Purchaser and Seller in proportion to their respective then-current Percentage Interests. All costs and expenses (including attorneys' fees and expenses) of any enforcement pursuant to this Section 5.10 (License Agreement) will be borne by [***] (subject to reimbursement from any Proceeds in accordance with the foregoing sentence). Nothing contained herein will limit Purchaser from retaining, at its sole cost, separate outside counsel who will be permitted, where reasonably practical, to consult with the lead counsel selected by Seller in accordance with Section 5.10.2 (License Agreement) for such enforcement.

5.10.4. Following the termination of any licenses to the Licensed Products pursuant to Section 13.2 (Termination) of the License Agreement prior to the Cap Date, Seller shall use commercially reasonable efforts to enter into one or more new licenses or other commercial arrangements for the purpose of Exploiting the Licensed Products. If Seller does not commence a replacement commercial relationship on financial terms consistent with those terms of the License Agreement within one hundred eighty (180) days of such termination and has not, commenced its own Exploitation of the Licensed Products, then Purchaser may, on behalf of Seller and with the cooperation of Seller seek and obtain on behalf of Seller such replacement commercial relationship. Following entry by Seller into any replacement commercial relationship, the term "License Agreement" herein shall reference the License Agreement and such replacement license agreement, and Purchaser and Seller shall take all reasonable efforts necessary or reasonably useful to amend the terms of this Agreement to account for the context of the replacement commercial relationship providing the Purchaser with the financial benefit bargained for in this Agreement.

5.11. **Listed Patent Rights**.

5.11.1. During the Purchased Receivable Period, Seller will, subject to the provisions of the License Agreement and any rights of Licensee thereunder (and, as between Licensee and Seller, to the extent Seller has such rights), (a) diligently, at its sole cost and expense as between the Parties, prosecute and maintain the Listed Patent Rights in the Territory in accordance with, and, subject to, the License Agreement, (b) with respect to each family of related patent filings within the Listed Patent Rights, if there is not an issued valid claim within a particular patent family covering the manufacture, use, import, offering for sale, or sale of the Licensed Products in a particular country in the U.S. or E.U. where there is a pending

application as of the Execution Date, then Seller will use diligent efforts, subject to its reasonable business judgment, to prosecute and obtain an issued valid claim covering the manufacture, use, import, offering for sale, or sale of the Product in such country, and (c) not disclaim or abandon any of the Listed Patent Rights in the Territory, or fail to take any action necessary to prevent the disclaimer or abandonment of the Listed Patent Rights in the Territory, except, in each case, where the disclaimer or abandonment of any such Listed Patent Rights is commercially reasonable. Subject to, and to the extent permitted under, the provisions of the License Agreement, Seller will diligently pursue, request, and seek to obtain term extensions of the Listed Patent Rights in each country of the Territory where permissible.

5.11.2. During the Purchased Receivable Period and subject to the provisions of the License Agreement and any rights of Licensee thereunder (and, as between Licensee and Seller, to the extent Seller has such rights), Seller will diligently, at its sole cost and expense (a) defend the Listed Patent Rights against any claims of invalidity or unenforceability in the Territory and (b) enforce the Listed Patent Rights (or cooperate with Licensee in connection with enforcement of the Listed Patent Rights, as applicable) against infringement, in each case, in any relevant jurisdiction in the Territory.

5.11.3. All costs and expenses (including attorneys' fees and expenses) incurred in connection with the prosecution, maintenance, defense, or enforcement of the Listed Patent Rights under this Section 5.11 (Listed Patent Rights) or under Article 12 (Intellectual Property) of the License Agreement will be borne by [***].

5.11.4. During the Purchased Receivable Period, the Proceeds (if any) of any enforcement of the Listed Patent Rights in the Territory brought pursuant to Section 12.5 (Patent Enforcement) of the License Agreement will be allocated between Seller and Purchaser in proportion to their respective Percentage Interests. The Proceeds (if any) of any enforcement of the Listed Patent Rights in the Territory brought pursuant to Section 12.5.2 (First Right and Step-In for Product Infringement) of the License Agreement will be allocated between Seller and Purchaser as follows: (a) each Party will be reimbursed for their unreimbursed costs and expenses incurred in the action, and (b) as to damages, Seller and Purchaser will share, in proportion to their respective Percentage Interests, an amount equal to a reasonable approximation of the royalties Licensee would have paid to Seller if Licensee had sold the infringing products and services rather than the infringer, *less* any Existing In-License Payments, with any balance thereof to be retained by Seller.

5.12. **Termination of the License Agreement**. Promptly, and in any event within 10 Business Days, following a Responsible Seller Party becoming aware of the occurrence of any event that gives rise to a right on the part of Seller to terminate the License Agreement in a country in the Territory during the Purchased Receivable Period, Seller will provide notice of such occurrence to Purchaser (to the extent such notice would not constitute a Confidentiality Breach) and consult with Purchaser in determining whether or not to exercise Seller's right to terminate the License Agreement pursuant to the applicable

section of the License Agreement. In any event, during the Purchased Receivable Period, Seller will not exercise its right to terminate the License Agreement with respect to any country in the Territory, except with the prior written consent of Purchaser.

5.13. **Tax Matters**.

5.13.1. All payments to any Party under this Agreement will be made without any deduction or withholding for or on account of any Tax, except as required by Applicable Law (a Party receiving such payment, the "**Withheld Party**"). If at any time a Party making a payment under this Agreement (the "**Withholding Party**") reasonably determines that deduction or withholding of any Tax is required from any such payment under this Agreement under Applicable Law, then (a) the Withholding Party will provide notice to the Withheld Party at least five Business Days prior to making such deduction or withholding, (b) the Withholding Party will timely remit the amount deducted or withheld to the applicable taxing authorities in accordance with Applicable Law, (c) such amounts remitted to the applicable taxing authorities will be deducted from the amounts otherwise payable to the Withheld Party by the Withholding Party prior to remittance to the Withheld Party, (d) if any such withholding or deduction by or on behalf of Seller is in respect of any Indemnified Tax, then Seller shall make an additional payment to Purchaser so that, after all such required deductions and withholdings are made by or on behalf of Seller in respect of such Indemnified Tax (including any such deductions and withholdings required with respect to any additional payments under this Section 5.13.1), Purchaser receives an amount equal to the amount that it would have received had no withholding of such Indemnified Tax been made, and (e) the Withholding Party will furnish to the Withheld Party proper evidence of the Taxes so remitted. Except with respect to withholding in respect of Indemnified Taxes described in clauses (c) or (d) of the preceding sentence, all amounts withheld or deducted by a Withholding Party or by Licensee shall be treated as received by the Withheld Party for purposes of this Agreement. The Withholding Party will use reasonable best efforts to cooperate with the Withheld Party in claiming exemptions or reductions from or refunds or credits of such deductions or withholdings, including in the five Business Day period following the delivery of notice in Section 5.13.1(a).

5.13.2. Each Party agrees that if any documentation related to Taxes it previously delivered expires or becomes obsolete or inaccurate in any respect, then it shall update such documentation or promptly notify the other Party in writing of its legal ineligibility to do so.

5.14. **Acknowledgment by Purchaser; Limitation of Seller's Duties and Obligations**. Notwithstanding any provision to the contrary set forth in this Agreement (including other provisions of this Article 5 (Covenants)), nothing contained in this Agreement will obligate Seller to (a) take any action, or omit to take any action, that (i) would conflict with, violate, or cause a violation of, contravene, or cause a default under, the License Agreement or any material Applicable Law or any judgment or order binding upon, Seller, or (ii) would, or would involve any disclosure that would, result in the loss or waiver of any attorney-client

privilege available to Seller, *provided* that Seller will use its reasonable efforts to implement arrangements that would permit such action, omission, or disclosure while preserving such privilege; or (b) assign any Listed Patent Rights to Purchaser or any other Person.

ARTICLE 6
THE CLOSING

6.1. **Closing**. The Closing will take place remotely via the exchange of documents and signatures on the date on which Purchaser delivers (or causes to be delivered) payment of the Closing Price to Seller in accordance with Section 2.2.1 and Section 6.2.1 (the "**Closing Date**").

6.2. **Payment of Purchase Price**.

6.2.1. No later than 15 Business Days following the Execution Date, Purchaser will deliver (or cause to be delivered) payment of the Closing Price, less the Purchaser Expenses, to Seller by electronic funds transfer or wire transfer of immediately available funds to one or more accounts specified by Seller.

6.2.2. The Milestone Payment, if any, shall be delivered to Seller in accordance with Section 2.2 (Purchase Price).

6.3. **Bill of Sale**. At the Closing, upon confirmation of the receipt of the Closing Price, Seller will deliver to Purchaser a duly executed Bill of Sale evidencing the sale, transfer, assignment, and conveyance of the Purchased Receivables.

6.4. **Payment Direction and Confidentiality Letter**. At the Closing, Seller and Purchaser will deliver to Purchaser a duly executed Payment Direction and Confidentiality Letter instructing Licensee with respect to all payments in respect of the Purchased Receivables.

6.5. **Tax Forms**. At the Closing, (a) Purchaser will deliver to Seller, a valid and properly completed and executed IRS Form W-9 certifying that Purchaser is (i) a U.S. Person and (ii) classified as a partnership for U.S. federal income tax purposes and (b) Seller will deliver to Purchaser a valid, properly completed and executed Internal Revenue Service Form W-8BEN-E certifying its non-U.S. status and that it is a qualified resident of the Netherlands that is entitled to benefits under the U.S./Netherlands income tax treaty (the "**Seller W-8**"). At the request of either Seller or Licensee, Purchaser, to the extent it is then legally eligible to do so, shall deliver to Licensee an IRS Form W-9 as described in clause (a) above.

ARTICLE 7
INDEMNIFICATION

7.1. **Indemnification by Purchaser**. Purchaser agrees to indemnify Seller from and against, and will pay to each Seller Indemnified Party the amount of, any and all Losses (including attorneys' fees and expenses) awarded against or incurred by Seller or any of its respective trustees, directors, officers, employees, or agents (each, a "**Seller Indemnified Party**"),

whether or not involving a Third Party claim, demand, action, or proceeding, to the extent arising out of (a) any breach of any representation, warranty, or certification made by Purchaser in this Agreement, (b) any breach of or default under any covenant or agreement by Purchaser in this Agreement, or (c) any fees, expenses, costs, liabilities, or other amounts incurred or owed by Purchaser to any brokers, financial advisors, or comparable other Persons retained or employed by Purchaser in connection with the transactions contemplated by this Agreement; provided, however, that the foregoing will exclude any indemnification to any Seller Indemnified Party (i) to the extent resulting from the bad faith, gross negligence, or willful misconduct of such Seller Indemnified Party, (ii) to the extent resulting from the failure of Seller to perform any of its obligations under any of the Transaction Documents, or (iii) to the extent resulting from acts or omissions of Purchaser based upon the written instructions from any Seller Indemnified Party. Notwithstanding any provision to the contrary set forth in this Agreement, following the final determination of any amount owed from Purchaser, as Indemnifying Party, to Seller, as Indemnified Party, Purchaser may elect, at its sole discretion, to satisfy any such indemnification obligation by instructing the Depositary Bank to deduct the amount of such indemnification obligation amount against each subsequent quarterly Purchased Receivable Payment due to Purchaser and pay such amount to Seller in satisfaction of such indemnification obligation.

7.2. **Indemnification by Seller**. Seller agrees to indemnify Purchaser from and against, and will pay to each Purchaser Indemnified Party the amount of, any and all Losses (including attorneys' fees and expenses) awarded against or incurred by Purchaser or any of its respective trustees, partners, directors, officers, employees, or agents (each, a "**Purchaser Indemnified Party**"), whether or not involving a Third Party claim, demand, action, or proceeding, to the extent arising out of (a) any breach of any representation, warranty or certification made by Seller in this Agreement, the License Agreement, or any Existing In-License, (b) any breach of or default under any covenant or agreement by Seller in this Agreement, the License Agreement, or any Existing In-License, or (c) any fees, expenses, costs, liabilities, or other amounts incurred or owed by Seller to any brokers, financial advisors, or comparable other Persons retained or employed by Seller in connection with the transactions contemplated by this Agreement; provided, however, that the foregoing will exclude any indemnification to any Purchaser Indemnified Party (i) to the extent resulting from the bad faith, gross negligence, or willful misconduct of such Purchaser Indemnified Party, (ii) to the extent resulting from the failure of Purchaser to perform any of its obligations under the Transaction Documents, or (iii) to the extent resulting from acts or omissions of Seller based upon the written instructions from any Purchaser Indemnified Party.

7.3. **Procedures**.

7.3.1. **Third Party Claims**. If any claim, demand, action, or proceeding will be brought against any Indemnified Party by a Third Party in respect of which indemnity is to be sought against either Party pursuant to Section 7.1 (Indemnification by Purchaser) or Section 7.2 (Indemnification by Seller), the Indemnified Party will, promptly after receipt of notice of the commencement of any such claim, demand, action, or proceeding, notify the indemnifying Party in writing of the commencement of such claim, demand, action, or proceeding, enclosing a copy of

all papers served, if any; provided that the omission to so notify the indemnifying Party will not relieve the indemnifying Party from any liability that it may have to any Indemnified Party under Section 7.1 (Indemnification by Purchaser) or Section 7.2 (Indemnification by Seller) unless, and only to the extent that, the indemnifying Party is actually and materially prejudiced by such omission. If any such action is brought against an Indemnified Party by a Third Party and it notifies Purchaser of the commencement thereof in accordance with this Section 7.3 (Procedures), then the indemnifying Party will be entitled, at the indemnifying Party's sole cost and expense, to participate therein and, to the extent that it may wish, to assume the defense thereof, with counsel of the indemnifying Party's choice (*provided* that such counsel is reasonably acceptable to the Indemnified Party), and, after notice from the indemnifying Party to such Indemnified Party of its election so to assume the defense thereof, the indemnifying Party will not be liable to such Indemnified Party under this Article 7 (Indemnification) for any legal or other expenses subsequently incurred by such Indemnified Party in connection with the defense. In any such proceeding, an Indemnified Party will have the right to retain its own counsel, but the reasonable fees and expenses of such counsel will be at the expense of such Indemnified Party unless (a) the indemnifying Party and the Indemnified Party will have agreed in writing to the retention of such counsel or (b) the named parties to any such proceeding (including any impleaded parties) include both the indemnifying Party and an Indemnified Party and representation of both parties by the same counsel would be inappropriate due to actual or potential conflicts of interests between them based on the advice of counsel to the indemnifying party. It is agreed that the indemnifying Party will not, in connection with any proceeding or related proceedings in the same jurisdiction, be liable for the reasonable fees and expenses of more than one separate law firm (in addition to local counsel where necessary) for all such Indemnified Parties. Indemnifying Party will not be liable for any settlement of any proceeding effected without its written consent, such consent not to be unreasonably withheld, conditioned, or delayed. Indemnifying Party will not, without the prior written consent of the Indemnified Party, effect any settlement, compromise, or discharge of any claim or pending or threatened proceeding in respect of which such Indemnified Party is or could have been a party and indemnity could have been sought hereunder by such Indemnified Party, unless such settlement, compromise, or discharge, as the case may be, includes an unconditional written release of such Indemnified Party, in form and substance reasonably satisfactory to the Indemnified Party, from all liability on claims that are the subject matter of such claim or proceeding. The Indemnified Party and indemnifying Party will cooperate in the defense or prosecution of any such claim, demand, action, or proceeding, with such cooperation to include (i) the retention of and the provision to the indemnifying party of records and information that are reasonably relevant to such claim, demand, action, or proceeding and (ii) the making available of employees on a mutually convenient basis for providing additional information and explanation of any material provided hereunder.

7.3.2. **Other Claims**. In order for an Indemnified Party to be entitled to any indemnification under this Article 7 (Indemnification) in respect of Losses that do not arise out of or involve a Third Party claim, the Indemnified Party must notify

indemnifying Party promptly in writing (including in such notice a brief description of the claim for indemnification and the Loss, including damages sought or estimated, to the extent actually known or reasonably capable of estimation by the Indemnified Party); provided, however, that the failure to promptly provide such notice will not affect the indemnification provided under this Article 7 (Indemnification) except to the extent that indemnifying Party has been actually and materially prejudiced as a result of such failure. The Indemnifying Party will have 30 days from receipt of such notice of claim to dispute the claim and will reasonably cooperate and assist the Indemnified Party in determining the validity of the claim for indemnity. To the extent necessary to review such notice, the Indemnified Party shall allow the Indemnifying Party reasonable access during normal business hours to investigate the matter or circumstance alleged to give rise to the applicable claim, and whether and to what extent any amount is payable in respect of the claim. If the Indemnifying Party does not give notice to the Indemnified Party that it disputes such claim within 30 days after its receipt of the notice of claim, then the claim specified in such notice of claim will be conclusively deemed a Loss subject to indemnification hereunder. If the Indemnifying Party responds to such notice and agrees with it, then such claim shall be deemed finally determined. If the Indemnifying Party responds to such notice and disagrees with all or a portion of the claim, then the portion of the claim that is agreed shall be deemed finally determined and the Indemnified Party and Indemnifying Party shall negotiate in good faith for a period of 30 days from the date of such response to resolve such unresolved portion of such claim. Following such 30-day negotiation period, either Party may bring an action or proceeding in accordance with Section 9.10 (Jurisdiction; Venue). The unresolved portion of any such claim shall not be deemed finally determined until and unless either resolved in writing by the Indemnified Party and the Indemnifying Party or in accordance with Section 9.10 (Jurisdiction; Venue).

7.4. **Limitations**. Notwithstanding any provision to the contrary set forth in this Agreement or in any other Transaction Document, (a) in no event will any Seller Indemnified Party or Purchaser Indemnified Party have any liability for, or Losses be deemed to include, any special, indirect, incidental, multiple, consequential, punitive, or exemplary damages, loss of use, business interruption, or loss of business opportunity (it being agreed that "lost profits" or similar losses up to the Cap Amount are regarded as direct damages), whether in contract or tort, regardless of whether the other Party in advised, has reason to know, or in fact knows of the possibility of such damages suffered or incurred by any such Seller Indemnified Party or Purchaser Indemnified Party in connection with this Agreement any of the other Transaction Documents or any of the transactions contemplated hereby or thereby, other than any such damages of Losses resulting by any breach of Section 5.8.4 (Specific Enforcement), (b) Seller will not have any liability under Section 7.2 (Indemnification by Seller) in excess of the Cap Amount based on a Cap Multiplier of [***]% (the "**Indemnification Cap**"), and (c) except with respect to breaches of Section 5.8 (Confidentiality), Purchaser will not have any liability under Section 7.1 (Indemnification by Purchaser) in excess of an amount equal to the aggregate amount of Purchased Receivables actually received by Purchaser at such time. Notwithstanding the foregoing, the limitations set forth in this Section 7.4 (Limitations) will not apply to any

claim for indemnification hereunder in the case of actual fraud, intentional misrepresentation, intentional wrongful acts, intentional breach, bad faith, or willful misconduct. The Parties acknowledge and agree that (a) Purchaser's Losses, if any, for any indemnifiable events under this Agreement will typically include Losses for Purchased Receivables that Purchaser was entitled to receive in respect of its ownership of the Purchased Receivables but did not receive timely or at all due to such indemnifiable event and (b) subject to this Section 7.4 (Limitations), Purchaser will be entitled to make indemnification claims for all such missing or delayed Purchased Receivables that Purchaser was entitled to receive in respect of its ownership of the Purchased Receivables as Losses hereunder (which claims will be reviewed and assessed by the Parties in accordance with the procedures set forth in this Article 7 (Indemnification)). This Article 7 (Indemnification) shall not apply with respect to Taxes, other than any Taxes that represent Losses arising from any non-Tax claim.

7.5. **No Implied Representations or Warranties**. EXCEPT AS SPECIFICALLY PROVIDED IN ARTICLE 3 (REPRESENTATIONS OF SELLER), SELLER MAKES NO REPRESENTATIONS AND EXTENDS NO WARRANTIES OF ANY KIND, WHETHER EXPRESS OR IMPLIED, WITH RESPECT TO THE PURCHASED RECEIVABLES, THE LICENSE AGREEMENT, THE LISTED PATENT RIGHTS, OR THE PRODUCT, OR ANY OTHER MATTER RELATING THERETO, INCLUDING THE IMPLIED WARRANTIES OF MERCHANTABILITY AND FITNESS FOR A PARTICULAR PURPOSE, AND SELLER HEREBY EXPRESSLY DISCLAIMS ALL SUCH IMPLIED WARRANTIES. Purchaser acknowledges that Seller has assumed no responsibilities of any kind with respect to any act or omission of Licensee with respect to the Exploitation, design, development, or other activities of Licensee with respect to the Product.

7.6. **Exclusive Remedy**. Except in the case of actual fraud, intentional misrepresentation, intentional wrongful acts, intentional breach, bad faith, or willful misconduct and except as set forth in Section 5.8.4 (Specific Enforcement), the indemnification afforded by this Article 7 (Indemnification) will be the sole and exclusive remedy for any and all Losses awarded against or incurred or suffered by a Party in connection with the transactions contemplated by the Transaction Documents, including with respect to any breach of any representation or warranty made by a Party in any of the Transaction Documents or any certificate delivered by a Party to the other Party in writing pursuant to this Agreement or any breach of or default under any covenant or agreement by a Party pursuant to any Transaction Document.

ARTICLE 8
TERMINATION

8.1. **Termination Date.** This Agreement will terminate upon expiration of the Term (such date, the "**Termination Date**"). If any payments are required to be made by one of the Parties hereunder after the Termination Date, then the applicable provisions of this Agreement will remain in full force and effect until any and all such payments have been made in full, and (except as provided in Section 8.3 (Survival)) solely for that purpose.

8.2. **Effects of Termination**. Upon the occurrence of the effective date of termination of this Agreement, the Back-Up Security Interest will be automatically and irrevocably released, and the Purchaser will cooperate with Seller to evidence such release by executing, delivering, or filing terminations, releases, or discharges of any Financing Statements and the Back-Up Security Interest contemplated by Section 2.1.2 (Financing Statements) (and execute, deliver, or file any other termination or release documents in connection therewith or otherwise to evidence any such termination) and all rights, title, and interests, in, to and under the Purchased Receivables revert to Seller. At Sellers's election and upon Seller's request, Purchaser will return or destroy all tangible materials comprising, bearing, or containing any Confidential Information that is in Purchaser's or its Affiliates' possession or control and provide written certification of such return or destruction.

8.3. **Survival**. The following provisions will survive any termination of this Agreement pursuant to this Section 8.3 (Survival): Section 5.7 (Use of Names); Section 5.8 (Confidentiality); Article 1 (Defined Terms) (solely to the extent necessary to give effect to the surviving provisions under this Section 8.3 (Survival)), and the rights, obligations or claims of either party accruing prior to the expiration of the Term; Article 7 (Indemnification); and Article 9 (Miscellaneous).

ARTICLE 9
MISCELLANEOUS

9.1. **Survival of Representations and Warranties**. The representations and warranties set forth in this Agreement (other than the Seller Fundamental Representations and the Purchaser Fundamental Representations) (collectively, the "**Business Representations**") shall survive the Closing for a period of two (2) years after the Closing Date at which time all such representations and warranties shall then terminate and no claim shall thereafter be made by any Party in respect thereof. The representations and warranties set forth in (a) Section 3.1 (Existence; Good Standing), Section 3.2 (Authorization), Section 3.3 (Enforceability), Section 3.4 (No Conflicts), Section 3.7 (No Liens; Title to Purchased Receivables), Section 3.11 (Brokers' Fees), Section 3.15 (Intellectual Property Matters) and Section 3.16 (License Agreement) (collectively, the "**Seller Fundamental Representations**") and (b) Section 4.1 (Existence; Good Standing), Section 4.2 (Authorization), Section 4.3 (Enforceability), Section 4.4 (No Conflicts), and Section 4.8 (Broker's Fees) (collectively, the "**Purchaser Fundamental Representations**") shall survive the Closing until the date that is six (6) years after the Closing Date at which time all such representations and warranties shall then terminate and no claim shall thereafter be made by any Party in respect thereof. The covenants and agreements contained in this Agreement that are to be performed prior to the Closing shall survive only until the Closing at which time all such covenants and agreements shall then terminate, and thereafter no claim shall be made by any Party in respect thereof. The covenants and agreements contained in this Agreement that are to be performed (in whole or in part) after the Closing shall survive the Closing in accordance with their terms and until fully performed at which time each such covenant and agreement shall then terminate, and thereafter no claim shall be made by any Party in respect thereof. Notwithstanding any provision to the contrary herein, in the event notice of a claim for indemnification hereunder is given within the applicable survival period, the representation or warranty, covenant or agreement that is

the subject of such indemnification claim (whether or not formal legal action shall have been commenced based upon such claim) shall survive with respect to such claim until such claim is finally resolved in accordance with the terms of Article 7. The foregoing limitations shall not apply in the event of any fraud by any Party. Each of the Parties acknowledges and agrees that this Section 9.1 is expressly intended to limit or expand (as applicable) otherwise applicable statute of limitations under Applicable Law, and waives the statute of limitations under such Applicable Law to the extent such statute of limitations period exceeds or is less than the periods described in this Section 9.1.

9.2. **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 9.2 (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) two Business Days after being dispatched through a reputable courier service, or (c) seven days after mailing, if mailed by first class certified or registered airmail, postage prepaid, return receipt requested.

if to Seller, to:

uniQure biopharma B.V.
Paasheuvelweg 25A
1105 BP Amsterdam
The Netherlands
Attention: Chief Financial 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: Hannah H. England
Email: Hannah.England@ropesgray.com

if to Purchaser, to:

HEMB SPV, L.P. c/o
HealthCare Royalty Partners IV, L.P.
300 Atlantic Street, Suite 600
Stamford, CT 06901
Attention: Clarke B. Futch
Timothy Bryant
John Urquhart

Email: clarke.futch@hcrx.com
tim.bryant@hcrx.com
john.urquhart@hcrx.com

and

Sagard Holdings Manager LP
161 Bay Street, Suite 5000
Toronto, Ontario
M5J 2S1
Canada
Attention: Sacha Haque
Raja Manchanda
Philippe Savard
William McIsaac

Email: legalteam@sagardholdings.com
manchanda@sagardholdings.com
savard@sagardholdings.com
mcisaac@sagardholdings.com

with a copies to (which will not constitute notice):

Sidley Austin LLP
2850 Quarry Lake Drive, Suite 301
Baltimore, MD 21209
Attention: Asher Rubin
Adriana Tibbitts
Email: arubin@sidley.com
atibbitts@sidley.com

Mintz Levin Cohn Ferris Glovsky & Popeo PC
919 Third Avenue
New York, NY 10022
(212) 692-6755 (w)
Attention: Richard Gervase
Email: rgervase@mintz.com

9.3. **Successors and Assigns**. The provisions of this Agreement shall be binding upon and inure to the benefit of the Parties and their respective successors and assigns. The Seller shall not be entitled to assign any of its obligations and rights under this Agreement without the prior written consent of Purchaser, not to be unreasonably withheld, conditioned or delayed; provided, however, such consent shall not be required in connection with the merger or other business combination involving Seller or the assignment of substantially all of Seller's obligations and rights to which this Agreement relates, so long as, the Person with which Seller has merged or combined or that has acquired such assets of Seller has delivered evidence, in form and substance reasonably satisfactory to Purchaser, to

Purchaser that such Person has assumed all of Seller's obligations under the Transaction Documents; provided, further that Seller shall remain liable for, and such assignment shall not relieve Seller of, any obligations set forth in this Agreement. Purchaser may assign or delegate any of its rights and obligations under the Transaction Documents without restriction; provided that such transfer is not to a competitor of Seller or Licensee. Any purported assignment in violation of this Section 9.3 (Successors and Assigns) shall be null and void *ab initio* and of no effect.

9.4. **Independent Nature of Relationship**. The relationship between Seller and Purchaser is solely that of seller and purchaser, and neither Seller nor Purchaser has any fiduciary or other special relationship with the other Party or any of its Affiliates. Nothing contained herein or in any other Transaction Document will be deemed to constitute Seller and Purchaser as a partnership, an association, a joint venture or any other kind of entity or legal form. The Parties recognize and agree that each is operating as an independent contractor and not as an agent, partner or fiduciary of the other.

9.5. **No Personal Liability**. It is expressly understood and agreed by Seller and Purchaser that:

9.5.1. each of the representations, warranties, covenants, and agreements in the Transaction Documents made on the part of Seller is made by Seller and is not intended to be nor is a personal representation, warranty, covenant, or agreement of any other Person, including those Persons named in the definition of "Knowledge" with respect to Seller or any other Representative of Seller or Seller's Affiliates (the "**Non-Warranting Parties**");

9.5.2. other than Seller, no Person, including the Non-Warranting Parties, will have any liability whatsoever for breach of any representation, warranty, covenant, or agreement made in the Transaction Documents on the part of Seller or in respect of any claim or matter arising out of, relating to, or in connection with the Transaction Documents or the transactions contemplated thereby; and

9.5.3. the provisions of this Section 9.5 (No Personal Liability) are intended to benefit each and every one of the Non-Warranting Parties and will be enforceable by each and every one of them to the fullest extent permitted by Applicable Law.

9.6. **Third Party Beneficiaries**. Except to the extent otherwise contemplated by Section 9.5 (No Personal Liability), this Agreement is for the sole benefit of Seller and Purchaser and their respective permitted successors and assigns, and nothing herein expressed or implied will give or be construed to give to any Person, other than the Parties and such successors and assigns, any legal or equitable rights hereunder.

9.7. **Entire Agreement**. This Agreement, together with the Exhibits hereto (which are incorporated herein by reference) and the other Transaction Documents, constitute the entire agreement between the Parties with respect to the subject matter hereof and supersede all prior agreements, understandings and negotiations, both written and oral, between the Parties hereto with respect to the subject matter of this Agreement. No representation, inducement, promise, understanding, condition, or warranty not set forth

herein (or in the Exhibits hereto or the other Transaction Documents) has been made or relied upon by either Party. All express or implied agreements, promises, assurances, arrangements, representations, warranties, and understandings as to the subject matter hereof, whether oral or written, heretofore made are superseded by this Agreement.

9.8. **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.

9.9. **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.

9.10. **Jurisdiction; Venue**.

9.10.1. EACH OF THE PARTIES HERETO HEREBY IRREVOCABLY AND UNCONDITIONALLY SUBMITS, FOR ITSELF AND ITS RESPECTIVE PROPERTY AND ASSETS, TO THE EXCLUSIVE JURISDICTION OF ANY NEW YORK STATE COURT OR FEDERAL COURT OF THE UNITED STATES OF AMERICA SITTING IN NEW YORK COUNTY, NEW YORK, AND ANY APPELLATE COURT THEREOF, IN ANY ACTION OR PROCEEDING ARISING OUT OF OR RELATING TO THIS AGREEMENT, OR FOR RECOGNITION OR ENFORCEMENT OF ANY JUDGMENT IN RESPECT THEREOF, AND PURCHASER AND SELLER HEREBY IRREVOCABLY AND UNCONDITIONALLY AGREE THAT ALL CLAIMS IN RESPECT OF ANY SUCH ACTION OR PROCEEDING MAY BE HEARD AND DETERMINED IN ANY SUCH NEW YORK STATE COURT OR, TO THE FULLEST EXTENT PERMITTED BY APPLICABLE LAW, IN SUCH FEDERAL COURT. PURCHASER AND SELLER HEREBY AGREE THAT A FINAL JUDGMENT IN ANY SUCH ACTION OR PROCEEDING WILL BE CONCLUSIVE AND MAY BE ENFORCED IN OTHER JURISDICTIONS BY SUIT ON THE JUDGMENT OR IN ANY OTHER MANNER PROVIDED BY APPLICABLE LAW. EACH OF PURCHASER AND SELLER HEREBY SUBMITS TO THE EXCLUSIVE PERSONAL JURISDICTION AND VENUE OF SUCH NEW YORK STATE AND FEDERAL COURTS. PURCHASER AND SELLER AGREE, TO THE FULLEST EXTENT PERMITTED BY APPLICABLE LAW, THAT PROCESS MAY BE SERVED ON PURCHASER OR SELLER IN THE SAME MANNER THAT NOTICES MAY BE GIVEN PURSUANT TO SECTION 9.2 (NOTICES).

9.10.2. EACH OF THE PARTIES HERETO HEREBY IRREVOCABLY AND UNCONDITIONALLY WAIVES, TO THE FULLEST EXTENT IT MAY

LEGALLY AND EFFECTIVELY DO SO, ANY OBJECTION THAT IT MAY NOW OR HEREAFTER HAVE TO THE LAYING OF VENUE OF ANY ACTION OR PROCEEDING ARISING OUT OF OR RELATING TO THIS AGREEMENT IN ANY NEW YORK STATE OR FEDERAL COURT. EACH OF PURCHASER AND SELLER HEREBY IRREVOCABLY WAIVES, TO THE FULLEST EXTENT PERMITTED BY APPLICABLE LAW, THE DEFENSE OF AN INCONVENIENT FORUM TO THE MAINTENANCE OF SUCH ACTION OR PROCEEDING IN ANY SUCH COURT.

9.10.3. Each Party hereby jointly and severally waives any and all right to trial by jury in any action or proceeding relating to this Agreement or any other document delivered hereunder or in connection herewith, or any transaction arising from or connected to any of the foregoing. Each of the Parties represents that this waiver is knowingly, willingly, and voluntarily given.

9.11. **Severability**. If one or more provisions of this Agreement are held to be invalid, illegal, or unenforceable by a court, arbiter or Governmental Authority, in each case, of competent jurisdiction, then such invalidity, illegality, or unenforceability will not affect any other provision of this Agreement, which will remain in full force and effect, and the Parties will replace such invalid, illegal, or unenforceable provision with a new provision permitted by Applicable Law and having an economic effect as close as possible to the invalid, illegal, or unenforceable provision. Any provision of this Agreement held invalid, illegal, or unenforceable only in part or degree by a court of competent jurisdiction will remain in full force and effect to the extent not held invalid, illegal, or unenforceable.

9.12. **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.

9.13. **Amendments; No Waivers**. Neither this Agreement nor any term or provision hereof may be amended, supplemented, restated, waived, changed, or modified except with the written consent of the Parties. No failure or delay by either Party in exercising any right, power, or privilege hereunder will operate as a waiver thereof nor will any single or partial exercise thereof preclude any other or further exercise thereof or the exercise of any other right, power, or privilege. No notice to or demand on either Party in any case will entitle it to any notice or demand in similar or other circumstances. No course of dealing between the Parties hereto will be effective to amend, modify, supplement, or waive any provision of this Agreement.

9.14. **Cumulative Remedies**. The remedies herein provided are cumulative and not exclusive of any remedies provided by Applicable Law.

9.15. **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."

9.16. **Counterparts**. This Agreement may be executed in any number of counterparts, each of which executed counterparts will constitute an original, and all of which counterparts together will constitute one and the same instrument. Copies of executed counterparts transmitted by email with PDF attachment will be considered original executed counterparts.

Signature Pages Follow

IN WITNESS WHEREOF, the undersigned have caused this Agreement to be executed by their respective representatives thereunto duly authorized as of the Execution Date.

uniQure biopharma B.V.

By: /s/ Christian Klemt
Name: Christian Klemt
Title: Managing Director

HemB SPV, L.P.

By: HemB SPV GP, LLC, its General Partner

By: /s/ Clarke B. Futch
Name: Clarke B. Futch
Title: Authorized Person

EXHIBIT A
[***]

EXHIBIT B
[***]

EXHIBIT C
[***]

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
(Principal Executive Officer)
August 1, 2023

Exhibit 31.2

Certification of Chief Financial Officer

I, Christian Klemt, 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/ CHRISTIAN KLEMT

Christian Klemt
Chief Financial Officer
(Principal Financial Officer)
August 1, 2023

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, 2023, as filed with the Securities and Exchange Commission on the date hereof (the "Report"), the undersigned, Matthew Kapusta, Chief Executive Officer, and Christian Klemt, Chief Financial Officer of the Company, hereby certify, 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
(Principal Executive Officer)

August 1, 2023

By: /s/ CHRISTIAN KLEMT

Christian Klemt
Chief Financial Officer
(Principal Financial Officer)

August 1, 2023

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.