REFINITIV

DELTA REPORT

10-Q

QURE - UNIQURE N.V.

10-Q - MARCH 31, 2024 COMPARED TO 10-Q - SEPTEMBER 30, 2023

The following comparison report has been automatically generated

TOTAL DELTAS	2582
CHANGES	276
DELETIONS	1452
ADDITIONS	854

REFINITIV

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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 September 30, 2023 March 31, 2024

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 November 2, 2023 May 2, 2024, the registrant had 47,811,479 48,549,437 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, activities related to our reorganization, 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, 2023February 28, 2024 (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 September 30, 2023March 31, 2024, 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.

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Part I – FINANCIAL INFORMATION

Item 1. Financial Statements

uniQure N.V.

UNAUDITED CONSOLIDATED BALANCE SHEETS

	September 30, 2023	December 31, 2022	March 31, 2024	December 31, 2023
	(in thousands, except share and per share amounts)			
			(in thousands, except share and per share amounts)	
Current assets				
Cash and cash equivalents	$ 229,484	$ 228,012	$ 243,062	$ 241,360
Current investment securities	429,428	124,831	312,621	376,532
Accounts receivable and contract asset	1,644	102,376		
Inventories	15,654	6,924		
Accounts receivable			10,717	4,193
Inventories, net			7,672	12,024
Prepaid expenses	14,884	11,817	18,839	15,089
Other current assets and receivables	2,532	2,814	3,092	2,655
Total current assets	**693,626**	**476,774**	**596,003**	**651,853**
Non-current assets				
Property, plant and equipment, net of accumulated depreciation of $51.6 million as of September 30, 2023 and $44.1 million as of December 31, 2022	45,946	50,532		
Non-current investment securities	—	39,984		
Property, plant and equipment, net of accumulated depreciation of $57.8 million as of March 31, 2024 and $55.7 million as of December 31, 2023			44,554	46,548
Operating lease right-of-use assets	30,360	32,726	27,695	28,789
Intangible assets, net, including in-process research and development asset of $56.6 million as of September 30, 2023 and $57.3 million as of December 31, 2022	57,976	58,778		
Intangible assets, net, including in-process research and development asset of $57.8 million as of March 31, 2024 and $59.1 million as of December 31, 2023			59,111	60,481
Goodwill	25,273	25,581	25,795	26,379
Deferred tax assets, net	12,351	14,528	11,594	12,276
Other non-current assets	6,018	6,061	5,298	5,363
Total non-current assets	**177,924**	**228,190**	**174,047**	**179,836**
Total assets	**$ 871,550**	**$ 704,964**	**$ 770,050**	**$ 831,689**
Current liabilities				
Accounts payable	$ 5,584	$ 10,984	$ 5,231	$ 6,586
Accrued expenses and other current liabilities	28,427	30,571	22,658	30,534
Current portion of contingent consideration	26,708	25,982	27,587	28,211
Current portion of operating lease liabilities	7,888	8,382	7,997	8,344
Total current liabilities	**68,607**	**75,919**	**63,473**	**73,675**
Non-current liabilities				
Long-term debt	101,431	102,791	102,120	101,749
Liability from royalty financing agreement	383,711	—	405,398	394,241
Operating lease liabilities, net of current portion	28,977	31,719	26,983	28,316
Contingent consideration, net of current portion	14,030	9,334	14,625	14,795
Deferred tax liability, net	4,917	8,257	7,376	7,543
Other non-current liabilities	1,093	935	3,321	3,700
Total non-current liabilities	**534,159**	**153,036**	**559,823**	**550,344**

Total liabilities	**602,766**	**228,955**	**623,296**	**624,019**
Commitments and contingencies				
Shareholders' equity				
Ordinary shares, €0.05 par value: 80,000,000 shares authorized as of September 30, 2023 and December 31, 2022 and 47,810,291 and 46,968,032 ordinary shares issued and outstanding as of September 30, 2023 and December 31, 2022, respectively	2,883	2,838		
Ordinary shares, €0.05 par value: 80,000,000 shares authorized as of March 31, 2024 and December 31, 2023 and 48,492,357 and 47,833,830 ordinary shares issued and outstanding as of March 31, 2024 and December 31, 2023, respectively			2,919	2,883
Additional paid-in-capital	1,141,662	1,113,393	1,155,904	1,148,749
Accumulated other comprehensive loss	(58,558)	(58,291)	(56,042)	(53,553)
Accumulated deficit	(817,203)	(581,931)	(956,027)	(890,409)
Total shareholders' equity	**268,784**	**476,009**	**146,754**	**207,670**
Total liabilities and shareholders' equity	**$ 871,550**	**$ 704,964**	**$ 770,050**	**$ 831,689**

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

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uniQure N.V.

UNAUDITED CONSOLIDATED STATEMENTS OF OPERATIONS AND COMPREHENSIVE LOSS

	Three months ended September 30,		Nine months ended September 30,		Three months ended March 31,	
	2023	2022	2023	2022	2024	2023
	(in thousands, except share and per share amounts)		(in thousands, except share and per share amounts)		(in thousands, except share and per share amounts)	
License revenues	$ 497	$ —	$ 1,290	$ —	$ 1,202	$ —
Contract manufacturing revenues	349	—	6,596	—	3,990	4,937
Collaboration revenues	561	1,449	1,268	3,738	3,293	388
Total revenues	**1,407**	**1,449**	**9,154**	**3,738**	**8,485**	**5,325**
Operating expenses:						
Cost of license revenues					(150)	—
Cost of contract manufacturing revenues	(1,006)	(861)	(4,793)	(1,693)	(9,076)	(2,435)
Research and development expenses	(65,400)	(48,068)	(172,245)	(139,263)	(40,692)	(60,809)
Selling, general and administrative expenses	(18,074)	(13,324)	(57,103)	(36,802)	(13,937)	(17,848)

Total operating expenses	**(84,480)**	**(62,253)**	**(234,141)**	**(177,758)**	**(63,855)**	**(81,092)**
Other income	1,424	1,485	4,537	4,981	1,376	1,811
Other expense	(228)	(199)	(673)	(621)	(234)	(216)
Loss from operations	**(81,877)**	**(59,518)**	**(221,123)**	**(169,660)**	**(54,228)**	**(74,172)**
Interest income	7,495	39	12,393	117	6,508	1,669
Interest expense	(15,444)	(3,069)	(25,846)	(8,279)	(16,097)	(3,562)
Foreign currency gains / (losses), net	186	14,362	(1,809)	42,328		
Other non-operating gains, net	—	—	—	635		
Loss before income tax benefit	**$ (89,640)**	**$ (48,186)**	**$ (236,385)**	**$ (134,859)**		
Income tax benefit	69	329	1,113	1,263		
Foreign currency losses, net					(1,145)	(2,369)
Loss before income tax (expense) / benefit					**$ (64,962)**	**$ (78,434)**
Income tax (expense) / benefit					(656)	1,207
Net loss	**$ (89,571)**	**$ (47,857)**	**$ (235,272)**	**$ (133,596)**	**$ (65,618)**	**$ (77,227)**
Other comprehensive loss:						
Foreign currency translation adjustments	(6,118)	(25,370)	(267)	(64,144)		
Other comprehensive (loss) / income:						
Foreign currency translation (loss) / gains, net					(2,524)	5,797
Defined benefit pension gain, net of taxes					35	—
Total comprehensive loss	**$ (95,689)**	**$ (73,227)**	**$ (235,539)**	**$ (197,740)**	**$ (68,107)**	**$ (71,430)**
Basic and diluted net loss per ordinary share	(1.88)	(1.02)	(4.94)	(2.86)	(1.36)	(1.63)
Weighted average shares used in computing basic and diluted net loss per ordinary share	47,770,101	46,772,430	47,619,875	46,680,667	48,384,510	47,436,335

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

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uniQure N.V.

UNAUDITED CONSOLIDATED STATEMENTS OF SHAREHOLDERS' EQUITY
FOR THE THREE MONTHS ENDED SEPTEMBER 30, MARCH 31, 2024 AND 2023 AND 2022

	Ordinary shares		Additional paid-in	Accumulated other comprehensive	Accumulated	Total shareholders'
	No. of shares	Amount	capital	loss	deficit	equity
	(in thousands, except share and per share amounts)					
Balance at June 30, 2022	**46,684,583**	**$ 2,823**	**$ 1,092,176**	**$ (67,630)**	**$ (540,881)**	**$ 486,488**
Loss for the period	—	—	—	—	(47,857)	(47,857)
Other comprehensive loss	—	—	—	(25,370)	—	(25,370)
Exercises of share options	47,290	2	258	—	—	260

Restricted share units distributed during the period	79,821	4	(4)	—	—	—
Share-based compensation expense	—	—	7,608	—	—	7,608
Issuance of ordinary shares relating to employee stock purchase plan	3,415	1	40	—	—	41
Balance at September 30, 2022	**46,815,109**	**$ 2,830**	**$ 1,100,078**	**$ (93,000)**	**$ (588,738)**	**$ 421,170**
Balance at June 30, 2023	**47,702,331**	**$ 2,877**	**$ 1,130,515**	**$ (52,440)**	**$ (727,632)**	**$ 353,320**
Loss for the period	—	—	—	—	(89,571)	(89,571)
Other comprehensive loss	—	—	—	(6,118)	—	(6,118)
Exercises of share options	400	—	2	—	—	2
Restricted and performance share units distributed during the period	100,296	5	(5)	—	—	—
Share-based compensation expense	—	—	11,097	—	—	11,097
Issuance of ordinary shares relating to employee stock purchase plan	7,264	1	53	—	—	54
Balance at September 30, 2023	**47,810,291**	**$ 2,883**	**$ 1,141,662**	**$ (58,558)**	**$ (817,203)**	**$ 268,784**

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

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uniQure N.V.

UNAUDITED CONSOLIDATED STATEMENTS OF SHAREHOLDERS' EQUITY
FOR THE NINE MONTHS ENDED SEPTEMBER 30, 2023 AND 2022

	Ordinary shares		Additional paid-in capital	Accumulated other comprehensive loss	Accumulated deficit	Total shareholders' equity	Ordinary shares		Additional paid-in capital	Accumulated other comprehensive loss	Accumulated
	No. of shares	Amount					No. of shares	Amount			deficit
	(in thousands, except share and per share amounts)						(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**					
Balance at December 31, 2022							**46,968,032**	**$ 2,838**	**$1,113,393**	**$ (58,291)**	**$ (581,931**
Loss for the period	—	—	—	—	(133,596)	(133,596)	—	—	—	—	(77,227
Other comprehensive loss	—	—	—	(64,144)	—	(64,144)					
Other comprehensive income							—	—	—	5,797	—

Exercises of share options	119,884	6	707	—	—	713	10,055	1	86	—	—
Restricted and performance share units distributed during the period	387,285	21	(21)	—	—	—	566,091	30	(30)	—	—
Share-based compensation expense	—	—	22,290	—	—	22,290	—	—	8,061	—	—
Issuance of ordinary shares relating to employee stock purchase plan	9,305	1	130	—	—	131	2,495	—	44	—	—
Balance at September 30, 2022	**46,815,109**	**$ 2,830**	**$1,100,078**	**$ (93,000)**	**$ (588,738)**	**$ 421,170**					
Balance at March 31, 2023							**47,546,673**	**$ 2,869**	**$1,121,554**	**$ (52,494)**	**$ (659,158**
Balance at December 31, 2022	**46,968,032**	**$ 2,838**	**$1,113,393**	**$ (58,291)**	**$ (581,931)**	**$ 476,009**					
Balance at December 31, 2023							**47,833,830**	**$ 2,883**	**$1,148,749**	**$ (53,553)**	**$ (890,409**
Loss for the period	—	—	—	—	(235,272)	(235,272)	—	—	—	—	(65,618
Other comprehensive loss	—	—	—	(267)	—	(267)					
Exercises of share options	12,882	1	122	—	—	123					
Restricted and performance share units distributed during the period	817,107	43	(43)	—	—	—					
Other comprehensive loss, net							—	—	—	(2,489)	—
Restricted share units distributed during the period							658,527	36	(36)	—	—
Share-based compensation expense	—	—	28,052	—	—	28,052	—	—	7,191	—	—

Issuance of ordinary shares relating to employee stock purchase plan	12,270	1	138	—	—	139				
Balance at September 30, 2023	**47,810,291**	**$ 2,883**	**$1,141,662**	**$ (58,558)**	**$ (817,203)**	**$ 268,784**				
Balance at March 31, 2024							**48,492,357**	**$ 2,919**	**$1,155,904**	**$ (56,042)**	**$ (956,027**

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

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uniQure N.V.

UNAUDITED CONSOLIDATED STATEMENTS OF CASH FLOWS

	Nine months ended September 30,		Three months ended March 31,	
	2023	2022	2024	2023
	(in thousands)			
			(in thousands)	
Cash flows from operating activities				
Net loss	$ (235,272)	$ (133,596)	$ (65,618)	$ (77,227)
Adjustments to reconcile net loss to net cash used in operating activities:				
Depreciation and amortization expense	7,712	6,206		
Amortization of premium/discount on investment securities	(6,529)	-		
Depreciation and amortization			2,629	2,527
Amortization of discount on investment securities			(3,695)	(925)
Share-based compensation expense	28,052	22,290	7,191	8,061
Royalty financing agreement interest expense	14,940	-	12,362	-
Deferred tax income	(1,113)	(1,263)		
Changes in fair value of contingent consideration and derivative financial instrument, net	15,441	6,875		

Unrealized foreign exchange losses / (gains), net	3,119	(36,390)		
Deferred tax expense / (income)			656	(1,207)
Changes in fair value of contingent consideration			165	975
Unrealized foreign exchange losses, net			1,389	1,193
Other items, net	2,146	(581)	2,778	456
Changes in operating assets and liabilities:				
Accounts receivable and contract asset, prepaid expenses, and other current assets and receivables	96,551	41,992		
Accounts receivable, prepaid expenses, and other current assets and receivables			(10,417)	(936)
Inventories	(8,730)	(4,075)	2,236	(553)
Accounts payable	(4,784)	4,529	(701)	(1,661)
Accrued expenses, other liabilities, and operating leases	(6,007)	3,633	(9,550)	(9,005)
Contingent consideration milestone payment	(1,914)	-		
Net cash used in operating activities	(96,388)	(90,380)	(60,575)	(78,302)
Cash flows from investing activities				
Investment in investment securities	(366,439)	-		
Proceeds on maturity of investment securities	106,307	-		
Proceeds on maturity of debt securities			150,107	5,330
Investment in debt securities			(83,778)	-
Purchases of property, plant, and equipment	(5,116)	(12,622)	(2,344)	(2,342)
Acquisition of uniQure France SAS (formerly Corlieve Therapeutics SAS), net of cash acquired	-	(1,900)		
Net cash used in investing activities	(265,248)	(14,522)		
Net cash generated from investing activities			63,985	2,988
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	262	844	-	131
Contingent consideration milestone payment	(7,649)	-		
Net cash generated from financing activities	362,675	844	-	131
Currency effect on cash, cash equivalents and restricted cash	424	(11,876)	(1,725)	1,034
Net increase / (decrease) in cash, cash equivalents and restricted cash	1,463	(115,934)	1,685	(74,149)
Cash, cash equivalents and restricted cash at beginning of period	231,173	559,353	244,544	231,173
Cash, cash equivalents and restricted cash at the end of period	**$ 232,636**	**$ 443,419**	**$ 246,229**	**$ 157,024**
Cash and cash equivalents	$ 229,484	$ 440,313	$ 243,062	$ 153,851
Restricted cash related to leasehold and other deposits	3,152	3,106	3,167	3,173
Total cash, cash equivalents and restricted cash	**$ 232,636**	**$ 443,419**	**$ 246,229**	**$ 157,024**
Supplemental cash flow disclosures:				
Cash paid for interest	$ (12,996)	$ (6,410)	$ (4,761)	$ (3,057)
Non-cash decrease in accounts payables and accrued expenses and other current liabilities related to purchases of property, plant, and equipment	$ (995)	$ (1,766)	$ (577)	$ (753)

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

uniQure N.V.

NOTES TO CONSOLIDATED FINANCIAL STATEMENTS
(Unaudited)

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 Dutch 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 nine months ended September 30, 2023 March 31, 2024, are not necessarily indicative of the results to be expected for the full year ending December 31, 2023 December 31, 2024, 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 December 31, 2023 filed by the Company with the SEC on February 27, 2023 February 28, 2024 (the "Annual Report").

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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. period. Actual results could differ from those estimates.

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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 December 31, 2023, 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 nine three months ended September 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® March 31, 2024. 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 nine three months ended September 30, 2023 March 31, 2024, as compared to the recent accounting pronouncements described in Note 2.3.25 2.3.27 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 a commercialization and license agreement with CSL Behring Agreement with CSL (the "CSL Behring Agreement"), 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 $0.5 million and $1.3 million $1.2 million of royalty revenue in each of the three and nine months ended September 30, 2023March 31, 2024, compared to nil in the three and nine months ended September 30, 2022March 31, 2023. 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 December 31, 2023, the Company recorded accounts receivable of $2.2 million $4.0 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 collected in July 2023. manufacturing revenue and royalty revenue.

As of September 30, 2023March 31, 2024, the Company had accounts receivable of $1.6 million $10.6 million from CSL Behring related to collaboration services, contract manufacturing revenue and royalty revenue.

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4 Investment securities

The following tables summarize the Company's investments in sovereign debt as of September 30, 2023 March 31, 2024 and December 31, 2022 December 31, 2023:

	At September 30, 2023				At March 31, 2024			
	Amortized cost, as adjusted	Gross unrealized holding gains	Gross unrealized holding losses	Estimated fair value	Amortized cost	Gross unrealized holding gains	Gross unrealized holding losses	Estimated fair value

	At December 31, 2022				At December 31, 2023			
	Amortized cost, as adjusted	Gross unrealized holding gains	Gross unrealized holding losses	Estimated fair value	Amortized cost	Gross unrealized holding gains	Gross unrealized holding losses	Estimated fair value
	(in thousands)				(in thousands)			
Current investments:								
Government debt securities (held-to-maturity)	$ 429,428	$ —	$ (556)	$ 428,872	$312,621	$ 12	$ —	$312,633
Total	**$ 429,428**	**$ —**	**$ (556)**	**$ 428,872**	**$312,621**	**$ 12**	**$ —**	**$312,633**

	At December 31, 2022				At December 31, 2023			
	Amortized cost, as adjusted	Gross unrealized holding gains	Gross unrealized holding losses	Estimated fair value	Amortized cost	Gross unrealized holding gains	Gross unrealized holding losses	Estimated fair value
	(in thousands)				(in thousands)			
Current investments:								
Government debt securities (held-to-maturity)	$ 124,831	$ —	$ (283)	$ 124,548	$376,532	$ 139	$ —	$376,671
Non-current investments:								
Government debt securities (held-to-maturity)	39,984	—	(43)	39,941				
Total	**$ 164,815**	**$ —**	**$ (326)**	**$ 164,489**	**$376,532**	**$ 139**	**$ —**	**$376,671**

The Company invests in short-term U.S. and European government bonds with the highest investment credit rating. The U.S. and European government bonds are U.S. dollar and euro denominated, respectively.

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

5 Inventories, net

The following table summarizes the inventory inventories, net balances as of September 30, 2023 March 31, 2024 and December 31, 2022 December 31, 2023:

September 30, 2023	December 31, 2022	March 31, 2024	December 31, 2023

	(in thousands)			
				(in thousands)
Raw materials	$ 7,426	$ 3,584	$ 6,331	$ 7,157
Work in progress	6,597	1,874	1,341	4,109
Finished goods	1,631	1,466	—	758
Inventories	**$ 15,654**	**$ 6,924**	**$ 7,672**	**$ 12,024**

The Company recorded write downs to net realizable value of $1.8 million for the three months ended March 31, 2024 and nil for the same period in 2023. The costs are recognized as Cost of Contract Manufacturing Revenues. As at March 31, 2024, and December 31, 2023, the Company recorded an allowance for inventory of $2.7 million and $1.6 million, respectively.

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.

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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, other assets, accounts payable, accrued expenses and other current liabilities reflected in the consolidated balance sheets approximate their fair values due to their short-term maturities.

The following table sets forth the Company's assets and liabilities that are required to be measured at fair value on a recurring basis as of September 30, 2023, March 31, 2024 and December 31, 2022 December 31, 2023:

	Quoted prices in active markets (Level 1)	Significant other observable inputs (Level 2)	Significant unobservable inputs (Level 3)	Total	Classification in Consolidated balance sheets	Quoted prices in active markets (Level 1)	Significant other observable inputs (Level 2)	Significant unobservable inputs (Level 3)	Total	Classification i balance
	(in thousands)									
At December 31, 2022										
						(in thousands)				
At December 31, 2023										
Assets:										
Cash and cash equivalents	$ 228,012	$ —	$ —	$228,012	Cash and cash equivalents	$ 241,360	$ —	$ —	$241,360	Cash and cash e
Restricted cash	3,161	—	—	3,161	Other non-current assets	3,184	—	—	3,184	Other non-currer
Total assets	$ 231,173	$ —	$ —	$231,173		**$ 244,544**	**$ —**	**$ —**	**$244,544**	
Liabilities:										
Contingent consideration	—	—	35,316	35,316	Contingent consideration	—	—	43,006	43,006	Contingent cons
Consideration for post-acquisition services	—	—	297	297	Other non-current liabilities	—	—	457	457	Other non-curren
Total liabilities	**$ —**	**$ —**	**$ 35,613**	**$ 35,613**		$ —	$ —	**$ 43,463**	**$ 43,463**	
At September 30, 2023										
At March 31, 2024										
Assets:										
Cash and cash equivalents	$ 229,484	$ —	$ —	$229,484	Cash and cash equivalents	$ 243,062	$ —	$ —	$243,062	Cash and cash e
Restricted cash	3,152	—	—	3,152	Other non-current assets	3,167	—	—	3,167	Other non-currer
Total assets	$ 232,636	$ —	$ —	$232,636		**$ 246,229**	**$ —**	**$ —**	**$246,229**	
Liabilities:										
Contingent consideration	—	—	40,738	40,738	Contingent consideration	—	—	42,212	42,212	Contingent cons
Consideration for post-acquisition services	—	—	478	478	Other non-current liabilities	—	—	498	498	Other non-curren
Total liabilities	**$ —**	**$ —**	**$ 41,216**	**$ 41,216**		**$ —**	**$ —**	**$ 42,710**	**$ 42,710**	

Contingent consideration

The Company is required to pay up to EUR 178.8 million (or $189.1 million $193.0 million based on the foreign exchange rate on September 30, 2023 March 31, 2024) to the former shareholders of uniQure France SAS (formerly Corlieve Therapeutics SAS) upon the achievement of contractually defined milestones in connection with the Company's July 2021 acquisition of uniQure France SAS. In September 2023, a milestone payment of EUR 10.0 million ($10.6 million) was paid, of which EUR 8.9 million ($9.6 million) related to contingent consideration.

The fair value of the contingent consideration as of September 30, 2023 March 31, 2024 was $40.7 million $42.2 million (December 31, 2022: $35.3 million 2023: $43.0 million) using discount rates of approximately 15.0% 14.8% to 15.5% 15.6% (December 31, 2022: 14.0% 2023: 15.3% to 14.4% 15.6%). Following the clearance of an Investigational New Drug ("IND") application for AMT-260 in August 2023, the The Company increased assumes the probability of achieving a EUR 30.0 million 30.0 million ($31.7 32.4 million) milestone payment due to following the dosing of the first patient dosed in Phase I/II clinical trial from 66.0% to 100.0% be 100%. This also resulted in an increase of, the probability that AMT-260 may advance to late-stage development and commercialization. The Company recorded $14.2 million and $15.4 million in expenses related to the changes in the fair value of the contingent consideration in the three and nine months ended September 30, 2023, respectively, compared to $5.5 million and $7.5 million during the same period in 2022.

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If as of September 30, 2023 March 31, 2024 the Company had assumed a 100% likelihood of AMT-260 advancing into a Phase III clinical study, then the fair value of the contingent consideration would have increased to $72.0 million. $74.8 million. If as of September 30, 2023 March 31, 2024 the Company had assumed that it would discontinue development of the AMT-260 program, then the contingent consideration would be have been 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.

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The following table presents the changes in fair value of contingent consideration between December 31, 2022 December 31, 2023 and September 30, 2023 March 31, 2024:

	Amount of contingent consideration	Amount of contingent consideration
	2023	2024
	(in thousands)	(in thousands)
Balance at December 31, 2022	**$ 35,316**	
Balance at December 31, 2023		**$ 43,006**
Change in fair value (presented within research and development expenses)	15,441	165
Contingent consideration milestone payment	(9,563)	
Currency translation effects	(456)	(959)
Balance at September 30, 2023	**$ 40,738**	
Balance at March 31, 2024		**$ 42,212**

As of September 30, 2023 March 31, 2024, the Company classified $26.7 million $27.6 million (December 31, 2023: $28.2 million) of the total contingent consideration of $40.7 million $42.2 million (December 31, 2023: $43.0 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 September 30, 2023 March 31, 2024 and December 31, 2022 December 31, 2023.

7 Accrued expenses and other current liabilities

Accrued expenses and other current liabilities include the following items:

	September 30, 2023	December 31, 2022	March 31, 2024	December 31, 2023
	(in thousands)			
			(in thousands)	
Personnel related accruals and liabilities			$ 9,572	$16,263
Accruals for goods received from and services provided by vendors-not yet billed	$ 11,688	$ 11,120	11,881	12,834
Personnel related accruals and liabilities	16,244	17,201		
Liability owed to the Purchaser pursuant to the Royalty Financing Agreement	495	—	1,205	1,437
Accrued contract fulfillment costs and costs to obtain a contract	—	2,250		
Total	**$ 28,427**	**$ 30,571**	**$22,658**	**$30,534**

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").

The total principal outstanding under the 2023 Amended Facility is $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% or 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 $102.5 million $103.3 million as of September 30, 2023 March 31, 2024, compared to $103.8 million $102.9 million as of December 31, 2022 December 31, 2023, and is recorded net of discount and debt issuance costs. The foreign currency loss on the facility in the three and nine months ended September 30, 2023 March 31, 2024 was $3.0 million and $1.4 million $2.3 million, respectively, compared to a foreign currency loss gain of $6.6 million and $15.0 million $0.7 million during the same period in 2022. 2023.

Interest expense associated with the 2023 Amended Facility during the three and nine months ended September 30, 2023 March 31, 2024 was $3.7 million and $10.9 million, respectively, compared to $3.0 million and $8.1 million $3.6 million during the same period in 2022. 2023.

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 $871.6 million $770.1 million, less $1.0 million $6.8 million of cash and cash equivalents and other current assets held by the Company, $85.0 million and $87.7 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 September 30, 2023 March 31, 2024, 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 a royalty purchase agreement (the "Royalty Financing Agreement Agreement") with the Purchaser. HemB SPV, L.P. (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 the Company. 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, a pre-specified threshold, 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 a 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 $1.3 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" at their fair market value 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 the 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 between $14.6 million $12.1 million and $16.5 million $13.7 million of interest expense through the nine three months ended September 30, 2023 ($11.5 million and $13.0 million, respectively, March 31, 2024 (nil for the three months ended September 30, 2023 March 31, 2023) if it had used 12.0% or 13.5%, instead of the $14.9 million $12.4 million recorded in the nine months ended September 30, 2023 ($11.8 million recorded through the three months ended September 30, 2023 March 31, 2024 (nil for the three months ended March 31, 2023). The Company will prospectively update the effective interest rate at each reporting date based on updated projections.

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

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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 December 31, 2023 and September 30, 2023 March 31, 2024:

	Amount of liability (in thousands)	Amount of liability (in thousands)
Gross proceeds from royalty financing agreement on June 5, 2023	$ 375,000	
Debt issuance costs paid	(4,938)	
Balance as of December 31, 2023 (includes $1.4 million presented as "Accrued expenses and other current liabilities)		$ **395,678**
Royalty payments to Purchaser	(796)	(1,437)
Liability owed to the Purchaser (presented as "Accrued expense and other current liabilities")	(495)	(1,205)
Interest expense for the period June 5, 2023 to September 30, 2023	14,940	
Interest expense for the period		12,362
Liability related to the royalty financing agreement	**$ 383,711**	**$ 405,398**

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. 14,351,471.

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, as amended. 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 September 30,		Nine months ended September 30,		Three months ended March 31,	
	2023	2022	2023	2022	2024	2023
	(in thousands)		(in thousands)			
					(in thousands)	
Cost of manufacturing services revenue	$ 315	$ 120	$ 477	$ 268	$ 345	$ 24
Research and development	5,638	3,730	14,675	12,090	3,425	4,305
Selling, general and administrative	5,144	3,758	12,900	9,932	3,421	3,732
Total	**$ 11,097**	**$ 7,608**	**$ 28,052**	**$ 22,290**	**$ 7,191**	**$ 8,061**

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

	Three months ended September 30,		Nine months ended September 30,		Three months ended March 31,	
	2023	2022	2023	2022	2024	2023
	(in thousands)		(in thousands)		(in thousands)	
Award type/ESPP						
Share options	$ 3,497	$ 3,448	$ 10,256	$ 10,141	$ 2,913	$ 3,274
Restricted share units	5,293	3,957	15,311	11,354	4,370	4,630
Performance share units	2,299	197	2,461	777	(99)	150
Employee share purchase plan	8	6	24	18	7	7
Total	**$ 11,097**	**$ 7,608**	**$ 28,052**	**$ 22,290**	**$ 7,191**	**$ 8,061**

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As of September 30, 2023 March 31, 2024, the unrecognized share-based compensation expense related to unvested awards under the 2014 Plans were:

	Unrecognized share-based compensation expense	Weighted average remaining period for recognition	Unrecognized share-based compensation expense	Weighted average remaining period for recognition
	(in thousands)	(in years)	(in thousands)	(in years)
Award type				
Share options	$ 27,765	2.59	$ 20,743	2.56
Restricted share units	35,763	2.03	27,981	1.99
Performance share units	556	1.20	80	0.61
Total	**$ 64,084**	**2.27**	**$ 48,804**	**2.23**

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 Any options that vest 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 nine three months ended September 30, 2023 March 31, 2024:

	Options		Options	
	Number of ordinary shares	Weighted average exercise price	Number of ordinary shares	Weighted average exercise price
Outstanding at December 31, 2022	**4,237,917**	**$ 26.13**		
Outstanding at December 31, 2023			**4,974,030**	**23.25**
Granted	1,575,430	$ 18.69	930,900	$ 5.59
Forfeited	(257,880)	$ 22.82	(71,437)	$ 21.26
Expired	(131,026)	$ 43.95	(11,584)	$ 37.61
Exercised	(12,882)	$ 9.58		
Outstanding at September 30, 2023	**5,411,559**	**$ 23.73**		
Thereof, fully vested, and exercisable on September 30, 2023	2,826,806	$ 26.43		
Thereof, outstanding and expected to vest after September 30, 2023	2,584,753	$ 20.76		
Outstanding at March 31, 2024			**5,821,909**	**$ 20.42**
Thereof, fully vested, and exercisable on March 31, 2024			3,054,242	$ 25.70
Thereof, outstanding and expected to vest after March 31, 2024			2,767,667	$ 14.60

Outstanding and expected to vest after December 31, 2023	2,098,557	$ 23.38

Total weighted average grant date fair value of options issued during the period (in $ millions)	$ 17.1	$3.0
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 September 30,		Nine months ended September 30,		Three months ended March 31,	
Assumptions	**2023**	**2022**	**2023**	**2022**	**2024**	**2023**
Expected volatility	70%	70%	70%	70%	70%	70%
Expected terms	10 years	10 years	10 years	10 years	10 years	10 years
Risk free interest rate	3.71% - 4.00%	4.16%	3.71% - 4.10%	2.12% - 4.16%	4.32%	4.10%
Expected dividend yield	0%	0%	0%	0%	0%	0%

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RSUs

The following table summarizes the RSU activity for the nine three months ended September 30, 2023 March 31, 2024:

	RSUs		RSUs	
	Number of ordinary shares	**Weighted average grant-date fair value**	**Number of ordinary shares**	**Weighted average grant-date fair value**
Non-vested at December 31, 2022	**1,818,774**	**$ 20.46**		
Non-vested at December 31, 2023			**2,264,369**	**$ 18.07**
Granted	1,614,925	$ 18.90	1,160,800	$ 5.59
Vested	(722,977)	$ 22.57	(659,240)	$ 20.30
Forfeited	(224,402)	$ 19.59	(58,401)	$ 17.39
Non-vested at September 30, 2023	**2,486,320**	**$ 18.91**		
Non-vested at March 31, 2024			**2,707,528**	**$ 12.19**
Total weighted average grant date fair value of RSUs granted during the period (in $ millions)		$ 30.5		$ 6.5

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

PSUs

The following table summarizes the PSU activity for the nine three months ended September 30, 2023 March 31, 2024:

	PSUs	PSUs

	Number of ordinary shares	Weighted average grant-date fair value	Number of ordinary shares	Weighted average grant-date fair value
Non-vested at December 31, 2022	**400,690**	**$ 28.82**		
Vested	(94,510)	$ 30.65		
Non-vested at December 31, 2023			**222,550**	**$ 28.09**
Forfeited	(42,745)	$ 28.04	(17,030)	$ 27.12
Non-vested at September 30, 2023	**263,435**	**$ 28.29**		
Non-vested at March 31, 2024			**205,520**	**$ 27.99**

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 Earned 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. As of September 30, 2023 March 31, 2024, two milestones had been achieved. achieved and vested in either 2022 or 2023. Additionally, another milestone is two milestones are considered probable as of September 30, 2023 December 31, 2023 and March 31, 2024.

The ESPP

During the nine three months ended September 30, 2023 March 31, 2024, 12,270 nil ordinary shares were issued under the ESPP compared to 9,305 2,495 during the same period in 2022. 2023. As of September 30, 2023 March 31, 2024, 103,790 96,862 ordinary shares remain available for issuance under the ESPP compared to a total of 117,997 113,565 as of September 30, 2022 March 31, 2023.

11 Income taxes

The Company recorded $0.1 million and $1.1 million $0.7 million deferred tax expense in relation to its operations in the U.S. during the three months ended March 31, 2024. The Company recorded $1.2 million deferred tax benefit in relation to its operations in the U.S. and France during the three and nine months ended September 30, 2023, respectively. The Company recorded $0.3 million and $1.3 million deferred tax benefit in relation to its operations in the U.S. and France during the three and nine months ended September 30, 2022, respectively. March 31, 2023.

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The effective income tax rate of (0.1%) and (0.5%) 1.0% during the three and nine months ended September 30, 2023 March 31, 2024 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. Netherlands and a partial a valuation allowance against its net deferred tax assets in France. The effective income tax rate during the three and nine months ended September 30, 2022 March 31, 2023 was (0.7%) and (0.9% (1.5%), respectively, as the Company had recorded a valuation allowance against its net deferred tax assets in the Netherlands.

12 14 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 nine months ended September 30, 2023 March 31, 2024, 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 nine months ended September 30, 2023 March 31, 2024. 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 September 30, 2023 March 31, 2024 and September 30, 2022 March 31, 2023, respectively.

The potentially dilutive ordinary shares are summarized below:

	Three months ended September 30,		Nine months ended September 30,		Three months ended March 31,	
	2023	2022	2023	2022	2024	2023
Anti-dilutive ordinary share equivalents						
Stock options under 2014 Plans and previous plan	5,411,599	4,284,656	5,411,599	4,284,656	5,821,909	5,150,690
Non-vested RSUs and PSUs	2,749,755	2,328,151	2,749,755	2,328,151	2,913,048	2,772,146
ESPP	2,857	721	2,857	721	3,140	801
Total anti-dilutive ordinary share equivalents	**8,164,211**	**6,613,528**	**8,164,211**	**6,613,528**	**8,738,097**	**7,923,637**

13 Subsequent events

On October 5, 2023, the Company announced a reorganization plan (the "Reorganization") pursuant to which the Company's global workforce will be reduced by approximately 20%. As part of the Reorganization, more than half of the Company's research and technology projects will be discontinued and a research lab in Lexington, Massachusetts will be closed. The Company estimated that it will incur charges of approximately $2.3 million in connection with the reorganization related to cash expenditures for employee severance costs. The Company also anticipates that there could be costs associated with the lab to be closed in Lexington and associated fixed assets if the carrying value is determined to not be recoverable as of the cease-use date, which is expected to be later in the fourth quarter of 2023. The Company anticipates the costs associated with the reorganization will be incurred in the fourth quarter of 2023.

None.

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

The following Management's Discussion and Analysis of Financial Condition and Results of Operations ("MD&A") is intended to help the reader understand our results of operations and financial condition. This MD&A is provided as a supplement to, and should be read in conjunction with, our unaudited consolidated financial statements and the accompanying notes thereto and other disclosures included in this Quarterly Report on Form 10-Q, including the disclosures under Part II, Item 1A "Risk Factors," and our audited financial information and the notes thereto included in our Annual Report on Form 10-K (the "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 devastating 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 mesial temporal lobe epilepsy ("rTLE" MTLE") and Fabry disease. In November 2022 and February 2023, our Our internally developed HEMGENIX®, a gene therapy for the treatment of hemophilia B, was has been approved for commercialization by the United States Food and Drug Administration (the "FDA") in November 2022 and the European Medicines Agency ("EMA"), respectively. In June 2020, we agreed to in February 2023. The approval of HEMGENIX® follows more than a decade of research and clinical development, represents a major milestone in the field of gene therapy and ushers in a new treatment approach for patients living with hemophilia B. We 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®. its commercialization. We are manufacturing HEMGENIX® for CSL Behring and are entitled to specific milestone payments and royalties on net sales under of 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 product, a portion of the which we sold to a royalty rights due to us from CSL Behring under the Commercialization and License Agreement. In August acquisition company in 2023 the FDA cleared the Investigational New Drug ("IND") application in exchange for AMT-260, our gene therapy candidate for refractory mesial temporal lobe epilepsy ("MTLE"). On October 5, 2023, we announced a Reorganization (see definition below) of our research and technology programs. up-front cash.

We believe our validated technology platform and manufacturing capabilities provide us with distinct competitive advantages, including the potential to reduce development risk, cost, and time to market. We produce our Adeno-associated virus ("AAV") based adeno-associated virus-based gene therapies in our own facilities with a proprietary, commercial-scale, current good manufacturing practices compliant, ("GMP") -compliant manufacturing process. We believe our Lexington, Massachusetts-based facility is one of the world's leading, most versatile, gene therapy manufacturing facilities.

Business developments

Below is a summary of our recent significant business developments:*Recent Product Candidate Developments*

Reorganization Huntington's disease program (AMT-130)

On October 5, 2023, we announced that we are implementing a reorganization plan (the "Reorganization"). As a result of the Reorganization, we will be discontinuing investments in more than half of AMT-130 is our research, including AMT-210 for the treatment of Parkinson's disease, and technology projects. Following the Reorganization, we intend to prioritize advancing our clinical-stage programs, including AMT-130 novel gene therapy candidate for the treatment of Huntington's disease, AMT-260 which utilizes our proprietary, gene-silencing miQURE platform and incorporates an AAV vector carrying a miRNA specifically designed to silence the huntingtin gene and the potentially highly toxic exon 1 protein fragment.

We are currently conducting a multi-center randomized, controlled, and blinded Phase I/II clinical trial for AMT-130 in the treatment U.S. ("US study") as well as an open-label Phase Ib/II study in Europe with the same early-manifest criteria for Huntington's disease as the U.S. study ("European study"). We completed the enrollment of refractory mesial temporal lobe epilepsy, AMT-162 for all 26 patients in the treatment first two cohorts of SOD1 amyotrophic lateral sclerosis our US study in March 2022 and AMT-191 for the treatment enrollment of Fabry disease 13 patients in the two cohorts of our European study in June 2023.

In November 2023, we initiated patient dosing in a third cohort of up to proof 12 patients to further investigate both doses of concept. AMT-130 in combination with perioperative immunosuppression. Patient enrollment is ongoing in this cohort with a focus on evaluating near-term safety and tolerability.

As a result of the Reorganization, In June 2023, we will eliminate approximately 20% of our total workforce and close our research laboratory in Lexington, MA. We expect announced interim data, including up to complete the Reorganization by the end of fiscal year 2023 and expect to incur costs of approximately $2.3 million, consisting primarily of cash expenditures related to employee severance costs. We also anticipate that there could be costs associated with the lab to be closed in Lexington and associated fixed assets if the carrying value is determined to not be recoverable as of the cease-use date, which is expected to be later 24-month follow-up, from 26 patients enrolled in the

fourth quarter ongoing U.S. Phase I/II clinical trial of 2023. As part of AMT-130. In December 2023, we announced updated interim data, including up to 30-month follow-up from the Reorganization we will also consolidate all good manufacturing practices ("GMP") manufacturing into our Lexington, MA manufacturing facility and consolidate process and analytical development into our Amsterdam, Netherlands facility. 26 patients enrolled in the ongoing US study as well as the 13 patients enrolled in the European study.

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As a result We plan to initiate interactions with the FDA in the second quarter of 2024. The goal of these interactions is to define the future clinical and regulatory pathway for AMT-130 and is expected to include discussions of the significant reduction in research activities, Ricardo Dolmetsch, Ph.D., departed as Chief Scientific Officer, effective October 4, 2023. Dr. Dolmetsch will serve as a scientific consultant through data from the end of ongoing Phase I/II trials and the year. In connection with Dr. Dolmetsch's transition, potential to leverage robust natural history comparators and long-term clinical data from the Board of Directors of the Company have appointed Richard Porter, Ph.D., our current Chief Business Officer, to serve as the Company's Chief Business and Scientific Officer, effective as of October 5, 2023. Phase I/II studies.

Temporal lobe epilepsy program (AMT-260)

In August 2023, the FDA cleared the IND application for AMT-260, our gene therapy candidate for refractory MTLE. AMT-260 comprises an AAV9 vector that locally delivers two engineered micro ribonucleic acids ("miRNAs") designed to degrade the GRIK2 gene and suppress the aberrant expression of glutamate receptor subtype GLUK2 that is believed to trigger seizures in patients with refractory MTLE. We acquired AMT-260 as part of our acquisition of uniQure France SAS (formerly Corlieve Therapeutics) in July 2021.

In September 2023, following We are initiating a Phase I/IIa clinical trial that will be conducted in the IND clearance, we paid EUR 10.0 million ($10.6 million) United States and consist of two parts. The first part is a multicenter, open-label trial with two dosing cohorts of six patients each to the former shareholders assess safety, tolerability, and first signs for efficacy of uniQure France SAS (formerly Corlieve Therapeutics SAS), AMT-260 in patients with refractory MTLE. The second part is expected to settle be a milestone payment owed in relation randomized, controlled trial to our 2021 acquisition. generate proof of concept ("POC") data.

Huntington's Fabry disease program (AMT-130) (AMT-191)

AMT-130AMT-191 is our novel gene therapy candidate for the treatment of Huntington's disease Fabry disease. AMT-191 comprises of an AAV5 capsid that incorporates the utilizes our α-galactosidase A ("GLA") transgene and a proprietary, gene-silencing miQURE platform and incorporates an AAV vector carrying a miRNA specifically designed to silence the huntingtin gene and the potentially highly toxic exon 1 protein fragment.

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 seven patients in the high-dose cohort. All 13 patients enrolled in the EU study received AMT-130. Two remaining patients initially scheduled for the EU open-label study will now be enrolled in Cohort 3, which is expected to start enrolling in the fourth quarter of potent, liver-specific promoter. In November 2023 and will be comprised of up to 12 patients.

On March 21, 2022, we announced that we completed the enrollment of all 26 patients in FDA had cleared 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 IND application 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 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.

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Exploratory efficacy data

We believe early interim 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 demonstrated 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 demonstrated 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 demonstrated 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.

Exploratory 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 CSF 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 CSF 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 did not appear to be significantly different from each other or from the natural history.

We are initiating a first-in-human Phase I/IIa clinical trial that will be conducted in the United States. The multicenter, open-label clinical trial consists of two dose-escalating cohorts of up to three adult male patients each to assess safety, tolerability, and early signs of efficacy of AMT-191 in patients with Fabry disease. Three patients will be dosed in the initial dose. If no dose-limiting toxicology is identified, the dose will be escalated. If dose-limiting toxicology occurs in one of the three initial patients, three additional patients will be enrolled at the same dose level. If no additional patients in the cohort experience a dose-limiting toxicology, the dose will be escalated. Assessments will be made at three- and six-months post-treatment.

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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”) AMT-162 is our gene therapy candidate 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 IND application for APB-102 and has granted Orphan Drug and Fast Track designation. We expect to initiate patient screening in the fourth quarter of 2023 with the first patient dosing occurring in the first quarter of 2024. Mutations in the SOD1 gene of ALS account for approximately one-fifth of all inherited forms of this fatal disease. APB-102 AMT-162 is comprised of a recombinant AAVrh10 vector that expresses a 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. The FDA has cleared the IND application for AMT-162 and has granted Orphan Drug and Fast Track designation.

We made an initial cash payment in February 2023 are initiating a first-in-human Phase I/II clinical trial that will be a U.S.-based, multi-center, open-label trial consisting of $10.0 million to Apic Bio that was recognized as a research and development expense. We owe three cohorts with up to $43.0 million in milestone payments to Apic Bio if AMT-162 is approved for commercialization four patients each receiving a one-time intrathecal infusion with immunosuppression. Safety, tolerability and early signs of efficacy will be evaluated in the U.S. and Europe.

Fabry disease program (AMT-191)

AMT-191 is our novel gene therapy candidate for the treatment of Fabry disease. AMT-191 comprises of an AAV5 capsid that incorporates the α-galactosidase A ("GLA") transgene and a proprietary, highly potent, liver-specific promoter. We expect to submit an IND in the fourth quarter of 2023 and to begin patient dosing in 2024.

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 $1.3 billion, under the terms of the CSL Behring Agreement.

Hercules Amendment

On May 12, 2023 we and Hercules Capital, Inc. ("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.

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Investment in debt securities

In July and September 2023, we invested $272.0 million and EUR 87.0 million (or a total of $366.4 million as of the investment dates) of our cash and cash equivalents into short-term U.S. and European government bonds that are U.S. dollar and euro denominated, respectively. Our investment policy requires us to invest in bonds with the highest investment grade credit rating. As of September 30, 2023, the bonds have remaining maturities ranging from one to ten months. We classify these bonds as held-to-maturity.

Financial Overview

Key components of our results of operations include the following:

	Three months ended September 30,		Nine months ended September 30,		Three months ended March 31,	
	2023	2022	2023	2022	2024	2023
	(in thousands)		(in thousands)			
					(in thousands)	
Total revenues	$ 1,407	$ 1,449	$ 9,154	$ 3,738	$ 8,485	$ 5,325
Cost of license revenues					(150)	—
Cost of contract manufacturing revenues	(1,006)	(861)	(4,793)	(1,693)	(9,076)	(2,435)
Research and development expenses	(65,400)	(48,068)	(172,245)	(139,263)	(40,692)	(60,809)
Selling, general and administrative expenses	(18,074)	(13,324)	(57,103)	(36,802)	(13,937)	(17,848)
Net loss	(89,571)	(47,857)	(235,272)	(133,596)	(65,618)	(77,227)

As of September 30, 2023 March 31, 2024 and December 31, 2022 December 31, 2023, we had cash and cash equivalents and investment securities of $658.9 million $555.7 million and $392.8 million $617.9 million, respectively. We had a net loss of $89.6 million and $235.3 million $65.6 million in the three and nine months ended September 30, 2023 March 31, 2024, respectively, compared to net loss of $47.9 million and $133.6 million $77.2 million for the same period in 2022. 2023. As of September 30, 2023 March 31, 2024 and December 31, 2022 December 31, 2023, we had accumulated deficits of $817.2 million $956.0 million and $581.9 million $890.4 million, respectively.

Compared to the current period, we expect that our expenses will decline following the Reorganization, until which time we decide to advance one of our gene therapy product candidates into late stage clinical development.

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 nine three months ended September 30, 2023 March 31, 2024 or reasonably possible changes of our critical accounting estimates as of September 30, 2023 March 31, 2024 that could have had a material impact on our results of operations for the three and nine months ended September 30, 2023 March 31, 2024.

Cost of contract manufacturing

We entered into a development and commercial supply agreement with CSL Behring in June 2020. Since April 1, 2022, we 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"); candidates;

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- 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; and
- the timing of regulatory approvals.

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

Selling, general and administrative expenses

Our general and administrative expenses consist principally of employee, office, 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.

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Results of Operations

Comparison of the three months ended September 30, 2023 March 31, 2024 and 2022 2023

The following table presents a comparison of our results of operations for the three months ended September 30, 2023 March 31, 2024 and 2022 2023.

	Three months ended September 30,			Three months ended March 31,		
	2023	2022	2023 vs 2022	2024	2023	2024 vs 2023
	(in thousands)					
						(in thousands)
Total revenues	$ 1,407	$ 1,449	$ (42)	$ 8,485	$ 5,325	$ 3,160
Operating expenses:						
Cost of license revenues				(150)	—	(150)
Cost of contract manufacturing	(1,006)	(861)	(145)	(9,076)	(2,435)	(6,641)
Research and development expenses	(65,400)	(48,068)	(17,332)	(40,692)	(60,809)	20,117
Selling, general and administrative expenses	(18,074)	(13,324)	(4,750)	(13,937)	(17,848)	3,911
Total operating expenses	**(84,480)**	**(62,253)**	**(22,227)**	**(63,855)**	**(81,092)**	**17,237**
Other income	1,424	1,485	(61)	1,376	1,811	(435)
Other expense	(228)	(199)	(29)	(234)	(216)	(18)
Loss from operations	**(81,877)**	**(59,518)**	**(22,359)**	**(54,228)**	**(74,172)**	**19,944**
Other non-operating items, net	(7,763)	11,332	(19,095)	(10,734)	(4,262)	(6,472)
Net loss before income tax benefit	**$ (89,640)**	**$ (48,186)**	**$ (41,454)**			
Income tax benefit	69	329	(260)			
Net loss before income tax (expense) / benefit				**$(64,962)**	**$(78,434)**	**$ 13,472**
Income tax (expense) / benefit				(656)	1,207	(1,863)
Net loss	**$ (89,571)**	**$ (47,857)**	**$ (41,714)**	**$(65,618)**	**$(77,227)**	**$ 11,609**

Revenue Revenues

Our revenue revenues for the three months ended September 30, 2023 March 31, 2024 and 2022 was 2023 were as follows:

	Three months ended September 30,			Three months ended March 31,		
	2023	2022	2023 vs 2022	2024	2023	2024 vs 2023
	(in thousands)					

				(in thousands)		
License revenues	$ 497	$ —	$ 497	$1,202	$ —	$ 1,202
Contract manufacturing revenues	349	—	349	3,990	4,937	(947)
Collaboration revenues	561	1,449	(888)	3,293	388	2,905
Total revenues	**$ 1,407**	**$ 1,449**	**$ (42)**	**$8,485**	**$5,325**	**$ 3,160**

License revenues

We recognize license revenues from CSL Behring, related to royalty payments owed on HEMGENIX® sales, when earned. For the three months ended September 30, 2023, March 31, 2024, we recognized $0.5 million $1.2 million of license revenues (nil for the same period in 2022) 2023).

Contract manufacturing revenues

We recognize contract manufacturing revenues related to contract manufacturing HEMGENIX® for CSL Behring. Contract manufacturing revenues is realized when earned upon sales of HEMGENIX® drug product to CSL Behring. We recognized $0.3 million $4.0 million contract manufacturing revenues in the three months ended September 30, 2023March 31, 2024, compared to nil $4.9 million for the same period in 2022. We did not recognize such revenues in the three months ended September 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. 2023.

Collaboration revenues

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

We entered into For the three months ended March 31, 2024 and 2023 we recognized $3.3 million and $0.4 million of collaboration research, and license agreements with Bristol-Myers Squibb ("BMS") in 2015 which were terminated on February 21, 2023.
revenue for CSL Behring, respectively.

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For the three months ended September 30, 2023, we recognized $0.6 million of collaboration revenue for CSL Behring. For the three months ended September 30, 2022, we recognized $1.5 million of collaboration revenue for CSL Behring and BMS.

Cost of contract manufacturing

We incurred $1.0 million $9.1 million of cost of contract manufacturing related to the manufacture of HEMGENIX® in the three months ended September 30, 2023 March 31, 2024, compared to $0.9 million $2.4 million cost of contract manufacturing in the three months ended September 30, 2022 March 31, 2023. The increase in cost of $6.7 million in 2024 is primarily related to expensing costs previously capitalized as work in progress, including a write-down to lower net realizable value.

R&D expenses

R&D expenses for the three months ended September 30, 2023 March 31, 2024 were $65.4 million $40.7 million, compared to $48.1 million $60.8 million for the same period in 2022. 2023. 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 September 30,			Three months ended March 31,		
	2023	2022	2023 vs 2022	2024	2023	2024 vs 2023
	(in thousands)			(in thousands)		
Huntington's disease (AMT-130)				$ 2,426	$ 3,733	$ (1,307)
Temporal lobe epilepsy (AMT-260)	$ 4,662	$ 2,499	$ 2,163	2,254	4,320	(2,066)
Huntington's disease (AMT-130)	3,116	4,330	(1,214)			
Amyotrophic lateral sclerosis (AMT-162)	2,420	—	2,420	1,977	10,041	(8,064)
Fabry disease (AMT-191)	358	549	(191)	1,370	1,088	282
Programs in preclinical development and platform related expenses				449	1,885	(1,436)
Etranacogene dezaparvovec (AMT-060/061)	—	277	(277)	—	667	(667)
Programs in preclinical development and platform related expenses	3,104	886	2,218			
Total direct research and development expenses	**$ 13,660**	**$ 8,541**	**$ 5,119**	**$ 8,476**	**$21,734**	**$ (13,258)**
Employee and contractor-related expenses	19,431	15,553	3,878	16,923	18,703	(1,780)
Fair value changes related to contingent consideration	14,229	5,503	8,726			
Facility expenses	6,652	6,482	170	7,095	6,786	309
Share-based compensation expense	5,638	3,730	1,908	3,425	4,305	(880)
Other expenses				2,687	4,400	(1,713)
Disposables	4,694	6,012	(1,318)	1,921	3,906	(1,985)
Other expenses	1,096	2,247	(1,151)			
Fair value changes related to contingent consideration				165	975	(810)
Total other research and development expenses	**$ 51,740**	**$ 39,527**	**$ 12,213**	**$32,216**	**$39,075**	**$ (6,859)**
Total research and development expenses	**$ 65,400**	**$ 48,068**	**$ 17,332**	**$40,692**	**$60,809**	**$ (20,117)**

Direct research and development expenses

Temporal lobe epilepsy (AMT-260)

In the three months ended September 30, 2023 and September 30, 2022, we incurred costs of $4.7 million and $2.5 million respectively, for the preclinical development of AMT-260.

Huntington's disease (AMT-130)

In the three months ended September 30, 2023 March 31, 2024 and September 30, 2022 March 31, 2023, our we incurred costs of $2.4 million and $3.7 million respectively. 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 U.S. and in Europe. In the prior year, we had enrollment for the European clinical trial.

Temporal lobe epilepsy (AMT-260)

In the three months ended March 31, 2024 and March 31, 2023, we incurred costs of $2.3 million and $4.3 million respectively, for the development of AMT-260. In August 2023, the FDA cleared our IND application, and we started incurring costs for the preparation of a Phase I clinical trial.

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 September 30, 2023 March 31, 2024, we incurred $2.4 million $2.0 million of costs (nil to initiate a Phase I/II clinical trial in 2024. In the prior period), three months ended March 31, 2023 we incurred $10.0 million of expenses related to our preclinical activities for AMT-162. the transaction.

Fabry disease (AMT-191)

In the three months ended September 30, 2023 March 31, 2024 and September 30, 2022 March 31, 2023, we incurred costs of $0.4 million $1.3 million and $0.5 million $1.1 million, respectively, related to our preclinical development of AMT-191.

Etranacogene dezaparvovec (AMT-060/061)

We have incurred external In November 2023, the FDA cleared the IND application, and we started incurring additional 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. Prior to the transition of these activities to CSL Behring in December 2022, we managed the trials and incurred related expenses. Direct research and development expenses related to clinical development and other regulatory activities and commercialization expenses incurred in the three months ended September 30, 2023 and September 30, 2022 are presented net of reimbursements due from CSL Behring and include settlement amounts from the transition. preparation.

Preclinical programs & platform development

In the three months ended September 30, 2023, March 31, 2024 and September 30, 2022 March 31, 2023, we incurred $3.1 million $0.4 million and $0.9 million $1.9 million of costs, respectively, primarily related to our preclinical activities associated with product candidates for various other research programs and technology innovation projects.

Etranacogene dezaparvovec (AMT-060/061)

We transitioned activities related to the clinical trial and long-term follow-up of patients 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 March 31, 2024 and March 31, 2023 are presented net of reimbursements due from CSL Behring and include settlement amounts from the transition.

Other research & development expenses

- We incurred $19.4 million $16.9 million in personnel and contractor-related expenses in the three months ended September 30, 2023 March 31, 2024, compared to $15.6 million $18.7 million for the same period in 2022. 2023. The increase decrease was primarily related to the hiring of reduction in personnel and contractors to support our clinical-stage programs and manufacturing operations, as well as an increase a result of the restructuring that occurred in severance costs; October 2023;
- We incurred $14.2 million $7.1 million in operating expenses and depreciation expenses related to our rented facilities in Amsterdam and Lexington, Massachusetts in the three months ended March 31, 2024, compared to $6.8 million in the same period in 2023;
- We incurred $3.4 million in share-based compensation expenses in the three months ended March 31, 2024, compared to $4.3 million for the same period in 2023. The decrease was primarily a result of the reduction in expense from the restructuring that occurred in October 2023;
- We incurred $2.7 million of other expenses for the three months ended March 31, 2024, compared to $4.4 million for the same period in 2023;
- We incurred $1.9 million in disposable costs in the three months ended March 31, 2024, compared to $3.9 million for the same period in 2023. The decrease was primarily a result of the reduction in expense from the restructuring that occurred in October 2023; and
- We incurred $0.2 million of expenses in the three months ended September 30, 2023 March 31, 2024 related to an increase in the fair value of contingent consideration associated with the acquisition of uniQure France SAS, compared to $5.5 million an $1.0 million increase in fair value for the same period in 2022. The change was primarily due to the increase in the probability of making a future milestone payment related to the first patient dosed in the Phase I/II clinical trial of AMT-260;
- We incurred $6.7 million in operating expenses and depreciation expenses related to our rented facilities in Amsterdam and Lexington, Massachusetts in the three months ended September 30, 2023, compared to $6.5 million in the same period in 2022;
- We incurred $5.6 million in share-based compensation expenses in the three months ended September 30, 2023, compared to $3.7 million for the same period in 2022. The increase was primarily a result of recognizing compensation cost related to performance share units granted in December 2021, for which achievement of related performance conditions was deemed probable in the three months ended September 30, 2023. Compensation cost was not recognized in the three months ended September 30, 2022 as achievement of related performance conditions was not deemed probable at this time;
- We incurred $4.7 million in disposable costs in the three months ended September 30, 2023, compared to $6.0 million for the same period in 2022; and
- We incurred $1.1 million of other expenses for the three months ended September 30, 2023, compared to $2.2 million for the same period in 2022. 2023.

Selling, general and administrative expenses

Selling, general and administrative expenses for the three months ended September 30, 2023 March 31, 2024 were $18.1 million $13.9 million, compared to $13.3 million $17.8 million for the same period in 2022. 2023.

- We incurred $6.4 million $6.2 million in personnel and contractor-related expenses in the three months ended September 30, 2023 March 31, 2024, compared to $5.2 million $5.9 million in the same period in 2022; 2023;
- We incurred $5.1 million $3.4 million in share-based compensation expenses in the three months ended September 30, 2023 March 31, 2024, compared to $3.8 million $3.7 million in the same period in 2022. The increase was primarily a result of an increase in expense related to performance share units granted in 2021; 2023;

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- We incurred $2.8 million $2.0 million in professional fees in the three months ended September 30, 2023 March 31, 2024, compared to $1.6 million $3.2 million in the same period in 2022. 2023. We regularly incur accounting, audit and legal fees associated with operating as a public company; company. In the prior period, we incurred professional fees related to our global licensing agreement with Apic Bio; and
- We incurred $2.8 million $2.0 million in other operating expenses in the three months ended September 30, 2023 March 31, 2024, compared to $2.0 million $4.0 million in the same period in 2022. 2023. The $2.0 million decrease was primarily related to a decrease in intellectual property fees as well as a reduction of expenses for information technology.

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Other items, net

We recognized $1.4 million $1.3 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 September 30, 2023 March 31, 2024, compared to $1.3 million for the same period in 2022. 2023.

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 September 30, 2023 March 31, 2024 and 2022 2023 were as follows:

	Three months ended September 30,			Three months ended March 31,		
	2023	2022	2023 vs 2022	2024	2023	2024 vs 2023
	(in thousands)					

				(in thousands)		
Interest income	$ 7,495	$ 39	$ 7,456	$ 6,508	$ 1,669	$ 4,839
Interest expense	(15,444)	(3,069)	(12,375)	(16,097)	(3,562)	(12,535)
Foreign currency gains, net	186	14,362	(14,176)			
Total non-operating (expense) / income, net	**$ (7,763)**	$ **11,332**	$ **(19,095)**			
Foreign currency losses, net				(1,145)	(2,369)	1,224
Total non-operating expense, net				**$(10,734)**	$**(4,262)**	$ **(6,472)**

We recognize interest income associated with our cash and cash equivalents and investment securities. We recognized $7.5 million $6.5 million in interest income in the three months ended September 30, 2023 March 31, 2024, compared to $0.0 million $1.7 million in the same period in the prior year. Our interest income increased by $7.5 million $4.8 million due to the interest income earned on investment securities as well as cash on hand during the three months ended September 30, 2023 March 31, 2024.

We recognized $15.4 million $16.1 million in interest expense for the three months ended September 30, 2023 March 31, 2024 and $3.1 million $3.6 million for the three months ended September 30, 2022 March 31, 2023. Our interest expense in 2023 2024 increased due to an increase in market interest rates related to the Hercules debt as well as the recognition of $11.8 million of interest expense related to the Royalty Financing Agreement that we entered into in May 2023.

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 $0.2 million during the three months ended September 30, 2023, compared to a net gain of $14.4 million during the same period in 2022.

Income tax benefit

We recognized $0.1 million of deferred tax benefit in the three months ended September 30, 2023, and $0.3 million of deferred tax benefit for the same period in 2022.

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Comparison of the nine months ended September 30, 2023 and 2022

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

	Nine months ended September 30,		
	2023	2022	2023 vs 2022
	(in thousands)		
Total revenues	$ 9,154	$ 3,738	$ 5,416
Operating expenses:			
Cost of contract manufacturing revenues	(4,793)	(1,693)	(3,100)
Research and development expenses	(172,245)	(139,263)	(32,982)
Selling, general and administrative expenses	(57,103)	(36,802)	(20,301)
Total operating expenses	**(234,141)**	**(177,758)**	**(56,383)**
Other income	4,537	4,981	(444)
Other expense	(673)	(621)	(52)
Loss from operations	**(221,123)**	**(169,660)**	**(51,463)**
Non-operating items, net	(15,262)	34,801	(50,063)
Loss before income tax benefit	$ **(236,385)**	$ **(134,859)**	**(101,526)**

Income tax benefit	1,113	1,263	(150)
Net loss	**$ (235,272)**	**$ (133,596)**	**$ (101,676)**

Revenue

Our revenue for the nine months ended September 30, 2023 and 2022 was as follows:

	Nine months ended September 30,		
	2023	2022	2023 vs 2022
	(in thousands)		
License revenues	$ 1,290	$ —	$ 1,290
Contract manufacturing revenues	$ 6,596	$ —	$ 6,596
Collaboration revenues	1,268	3,738	(2,470)
Total revenues	**$ 9,154**	**$ 3,738**	**$ 5,416**

License revenue

We recognize license revenues from CSL Behring related to royalty payments owed on HEMGENIX® sales, when earned. For the nine months ended September 30, 2023, we recognized $1.3 million of license revenue (nil for the same period in 2022).

Contract manufacturing revenues

We recognize contract manufacturing revenue related to contract manufacturing HEMGENIX® drug product for CSL Behring. Contract manufacturing revenues is realized when earned upon sales of HEMGENIX®to CSL Behring.We recognized $6.6 million contract manufacturing revenues in the nine months ended September 30, 2023, compared to nil for the same period in 2022. We did not recognize such revenues in the nine months ended September 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 BMS in 2015 which were terminated on February 21, 2023.

For the nine months ended September 30, 2023, we recognized $1.3 million of collaboration revenue for CSL Behring. For the nine months ended September 30, 2022, we recognized $3.7 million of collaboration revenue for CSL Behring and BMS.

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Cost of contract manufacturing

We incurred $4.8 million of cost of contract manufacturing related to the manufacture of HEMGENIX® in the nine months ended September 30, 2023, compared to $1.7 million cost of contract manufacturing in the nine months ended September 30, 2022.

R&D expenses

R&D expenses for the nine months ended September 30, 2023 were $172.2 million, compared to $139.3 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.

	Nine months ended September 30,		
	2023	2022	2023 vs 2022
		(in thousands)	
Amyotrophic lateral sclerosis (AMT-162)	$ 13,048	$ —	$ 13,048
Temporal lobe epilepsy (AMT-260)	11,970	11,394	576
Huntington's disease (AMT-130)	11,093	15,721	(4,628)
Fabry disease (AMT-191)	1,846	1,664	182
Etranacogene dezaparvovec (AMT-060/061)	(1,336)	1,008	(2,344)
Programs in preclinical development and platform related expenses	7,246	3,919	3,327
Total direct research and development expenses	**$ 43,867**	**$ 33,706**	**$ 10,161**
Employee and contractor-related expenses	55,894	46,786	9,108
Facility expenses	21,114	17,150	3,964
Share-based compensation expense	14,675	12,090	2,585
Disposables	14,475	15,077	(602)
Fair value changes related to contingent consideration	15,441	7,510	7,931
Other expenses	6,779	6,944	(165)
Total other research and development expenses	**$ 128,378**	**$ 105,557**	**$ 22,821**
Total research and development expenses	**$ 172,245**	**$ 139,263**	**$ 32,982**

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 $13.0 million expenses recorded to research and development expense related to the acquisition, as well as for preclinical activities of AMT-162 during the nine-month period ended September 30, 2023.

Temporal lobe epilepsy (AMT-260)

In the nine months ended September 30, 2023 and September 30, 2022, we incurred costs of $12.0 million and $11.4 million, respectively, for the preclinical development of AMT-260.

Huntington's disease (AMT-130)

In the nine months ended September 30, 2023 and September 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.

Fabry disease (AMT-191)

In the nine months ended September 30, 2023 and September 30, 2022, we incurred costs of $1.8 million and $1.7 million, respectively, related to our preclinical development of AMT-191.

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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. Prior to the transition of these activities to CSL Behring in December 2022, we managed the trials and incurred related expenses. Direct research and development expenses related to clinical development and other regulatory activities and commercialization expenses incurred in the nine months ended September 30, 2023 and September 30, 2022 are presented net of reimbursements due from CSL Behring and include settlement amounts from the transition.

Preclinical programs & platform development

In the nine months ended September 30, 2023, and September 30, 2022, we incurred costs of $7.2 million and $3.9 million, 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 $55.9 million in personnel and contractor-related expenses in the nine months ended September 30, 2023, compared to $46.8 million for the same period in 2022. The increase was primarily related to the hiring of personnel and contractors to support our clinical-stage programs and manufacturing operations, as well as an increase in severance costs;
- We incurred $21.1 million in operating expenses and depreciation expenses related to our rented facilities in Amsterdam and Lexington, Massachusetts in the nine months ended September 30, 2023, compared to $17.2 million in the same period in 2022. The increase primarily related to operating expenses and depreciation expense incurred from additional sites in Lexington which commenced in May and November 2022;
- We incurred $15.4 million of expenses in the nine months ended September 30, 2023 related to an increase in the fair value of contingent consideration associated with the acquisition of uniQure France SAS, compared to $7.5 million for the same period in 2022. The change was primarily due to the increase in the probability of making a future milestone payment related to the first patient dosed in the Phase I/II clinical trial of AMT-260;
- We incurred $14.7 million in share-based compensation expenses in the nine months ended September 30, 2023, compared to $12.1 million for the same period in 2022. The increase was primarily a result of an increase in awards granted as well as from recognizing compensation cost related to performance share units granted in December 2021, for which achievement of related performance conditions was deemed probable in the nine months ended September 30, 2023. Compensation cost was not recognized in the nine months ended September 30, 2022 as achievement of related performance conditions was not deemed probable at this time;
- We incurred $14.5 million in disposable costs in the nine months ended September 30, 2023, compared to $15.1 million for the same period in 2022; and
- We incurred $6.8 million of other expenses for the nine months ended September 30, 2023, compared to $6.9 million for the same period in 2022. There was an increase related to contractual payments of $3.1 million which we owed to a licensor upon the Europeans Medicines Agency approval of HEMGENIX® in February 2023, which were offset by a reduction in consultant-related expenses.

Selling, general and administrative expenses

Selling, general and administrative expenses for the nine months ended September 30, 2023 were $57.1 million, compared to $36.8 million for the same period in 2022.

- We incurred $18.8 million in personnel and contractor-related expenses in the nine months ended September 30, 2023, compared to $14.8 million in the same period in 2022. The increase was primarily related to the hiring of personnel and contractors to support our business operations;
- We incurred $12.9 million in share-based compensation expenses in the nine months ended September 30, 2023, compared to $9.9 million in the same period in 2022. The increase was primarily a result of an increase in awards grants and from recognizing compensation cost for performance share units granted in December 2021 that were deemed probable in the nine months ended September 30, 2023;

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- We incurred $8.5 million in professional fees in the nine months ended September 30, 2023, compared to $4.3 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 and other corporate initiatives;

- We incurred $3.8 million in financial advisory fees in relation to our licensing transaction with CSL Behring in the nine months ended September 30, 2023 (nil in prior period);
- We incurred $3.3 million in intellectual property fees including registration and professional fees in the nine months ended September 30, 2023 compared to $1.2 million in the same period in 2022. The increase mainly related to an increase in professional fees; and
- We incurred $9.0 million in other operating expenses in the nine months ended September 30, 2023, compared to $6.0 million in the same period in 2022. The increase was primarily a result of an increase in information technology expenses.

Other items, net

We recognized $3.9 million in other income from payments received from European authorities to subsidize our research and development efforts in the Netherlands in the nine months ended September 30, 2023, compared to $3.9 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 nine months ended September 30, 2023 and 2022 were as follows:

	Nine months ended September 30,		
	2023	2022	2023 vs 2022
	(in thousands)		
Interest income	$ 12,393	$ 117	$ 12,276
Interest expense	(25,846)	(8,279)	(17,567)
Foreign currency (losses) / gains, net	(1,809)	42,328	(44,137)
Other non-operating gains	—	635	(635)
Total non-operating (expense) / income, net	**$ (15,262)**	**$ 34,801**	**$ (50,063)**

We recognize interest income associated with our cash and cash equivalents and investment securities. We recognized $12.4 million in interest income in the nine months ended September 30, 2023, compared to $0.1 million in the same period in the prior year. Our interest income increased by $12.3 million due to the interest income earned on investment securities as well as cash on hand during the nine months ended September 30, 2023.

We recognized $25.8 million in interest expense for the nine months ended September 30, 2023 and $8.3 million for the nine months ended September 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 $14.9 million of interest expense related to the Royalty Financing Agreement that we entered into in May 2023.

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 $1.8 million $1.1 million during the nine three months ended September 30, 2023 March 31, 2024, compared to a net gain loss of $42.3 million $2.4 million during the same period in 2022. 2023.

Income tax benefit

We recognized $1.1 million $0.7 million of deferred tax benefit expense in the nine three months ended September 30, 2023March 31, 2024, and $1.3 million $1.2 million of deferred tax benefit for the same period in 2022. 2023.

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Financial Position, Liquidity and Capital Resources

As of September 30, 2023, March 31, 2024, we had cash and cash equivalents, restricted cash and investment securities of $662.1 million. Until $558.9 million. Until such time, if ever, as we can generate substantial cash flows from successfully commercializing our proprietary product candidates, 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. Based on our current operating plan, research and development plans and our timing expectations related to the progress of our programs and following the Reorganization, we believe that our cash and cash equivalents and investment securities will fund our operations into the second quarter of 2027. We have based this estimate on assumptions that may prove to be wrong, and we could exhaust our available capital resources sooner than we expect. We expect that we will require additional funding if we decide to advance AMT-130 for our Huntington's disease gene therapy program or any of our other product candidates into late stage late-stage clinical development. Our material cash requirements include the following contractual and other obligations:

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Debt

As of September 30, 2023 March 31, 2024, 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 $50.9 million $44.2 million, with $13.4 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 September 30, 2023 March 31, 2024, we had fixed lease payment obligations of $54.8 million $50.7 million, with $8.3 million $8.4 million payable within 12 months.

Commitments related to uniQure France SAS acquisition (nominal amounts)

In relation to the our acquisition of uniQure France SAS acquisition, in 2021, 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. In September 2023, we made a payment of EUR 10.0 million ($10.6 million) to the former shareholders of uniQure France SAS following the FDA's clearance of the IND application for AMT-260. As of September 30, 2023 March 31, 2024, our remaining commitment amounts include a EUR 30.0 million ($31.7 32.4 million) milestone payment due upon treating the first patient in a Phase I/II clinical trial for AMT-260 and EUR 160.0 million ($169.2 172.7 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 September 30, 2023 March 31, 2024, of

$1.06/$1.08/€1.00. As of September 30, 2023 March 31, 2024, we expect these obligations will become payable between early 2024 and 2031. If and when due, up to 25% of the milestone payments can be settled with our ordinary shares.

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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 as a result of collecting payments related to our sale of the exclusive global rights to the Product of HEMGENIX® 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 nine three months ended:

	Nine months ended September 30,		Three months ended March 31,	
	2023	2022	2024	2023
	(in thousands)		(in thousands)	
Cash, cash equivalents and restricted cash at the beginning of the period	$ 231,173	$ 559,353	$ 244,544	$ 231,173
Net cash used in operating activities	(96,388)	(90,380)	(60,575)	(78,302)
Net cash used in investing activities	(265,248)	(14,522)		
Net cash generated from investing activities			63,985	2,988
Net cash generated from financing activities	362,675	844	—	131
Foreign exchange impact	424	(11,876)	(1,725)	1,034
Cash, cash equivalents and restricted cash at the end of period	**$ 232,636**	$ **443,419**	**$ 246,229**	$ **157,024**

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 with the exception of generating income in 2021 after receiving the upfront payment upon closing of the CSL Behring Agreement in 2021. Agreement. We continue to incur losses in the current period. We recorded a net loss of $89.6 million and $235.3 million $65.6 million in the three and nine months ended September 30, 2023 March 31, 2024, respectively, compared to a net loss of $47.9 million and $133.6 million $77.2 million during the same period in 2022. 2023. As of September 30, 2023 March 31, 2024, we had an accumulated deficit of $817.2 million $956.0 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 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% or 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 cash used in operating activities

Net cash used in operating activities was $96.4 million $60.6 million for the nine three months ended September 30, 2023 March 31, 2024 and consisted of net loss of $235.3 million $65.6 million adjusted for non-cash items, including depreciation and amortization expense of $7.7 million $2.6 million, amortization of the premium/discount on investment securities of $6.5 million $3.7 million, share-based compensation expense of $28.1 million $7.2 million, changes in the fair value of contingent consideration of $15.4 million $0.2 million, unrealized foreign exchange losses of $3.1 million $1.4 million, $14.9 million $12.4 million of interest expense related to the royalty financing and a change in deferred taxes of $1.1 million $0.7 million. Net cash used in operating activities also included favorable unfavorable changes in operating assets and liabilities of $77.0 million $18.4 million. There was a net decrease increase in accounts receivable, prepaid expenses, and other current assets and receivables of $96.6 million, primarily related to the collection of the $100.0 million milestone due from CSL Behring in July 2023$10.4 million. There was an increase a decrease in inventory balances of $8.7 million $2.2 million. There was a net decrease in accounts payable, accrued expenses, other liabilities, and operating leases of $10.8 million $10.3 million, primarily related to a decrease of $4.8 million in accounts payable and a decrease of $6.0 million $6.7 million from personnel related to various accruals. Net cash used in operating activities also includes a payment for a contingent consideration milestone of $1.9 million.

Net cash used in operating activities was $90.4 million $78.3 million for the nine three months ended September 30, 2022 March 31, 2023 and consisted of a net loss of $133.6 million $77.2 million adjusted for non-cash items, including depreciation and amortization expense of $6.2 million $2.5 million, amortization of the discount on investment securities of $0.9 million, share-based compensation expense of $22.3 million $8.1 million, changes in the fair value of contingent consideration and the derivative financial liability of $6.9 million $1.0 million, unrealized foreign exchange gains losses of $36.4 million $1.2 million and a change in deferred taxes of $1.3 million $1.2 million. Net cash generated from operating activities also included favorable unfavorable changes in operating assets and liabilities of $46.1 million $12.2 million. There was a net decrease increase in accounts receivable, and contract asset, prepaid expenses, and other current assets and receivables of $42.0 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. There was an increase in inventories of $4.1 million related to the production of etranacogene dezaparvovec under the CSL Behring Agreement. $0.9 million. These changes also relate to a net increase decrease in accounts payable, accrued expenses, other liabilities, and operating leases of $8.2 million $10.7 million, primarily related to increase in accounts payable. a decrease of $7.9 million from personnel related accruals.

Net cash used in investing activities

In the nine months ended September 30, 2023, we used $265.2 million in our investing activities compared to using $14.5 million for the same period in 2022.

	Nine months ended September 30,	
	2023	2022
	(in thousands)	
Investment in investment securities	$ (366,439)	$ —
Proceeds from maturity of investment securities	106,307	—
Build out of Amsterdam site	(2,161)	(8,265)
Build out of Lexington site	(2,955)	(4,357)
Acquisition of uniQure France SAS, net of cash acquired	—	(1,900)
Net cash used in investing activities	**$ (265,248)**	**$ (14,522)**

During the nine months ended September 30, 2023, we invested $366.4 million of our cash on hand into euro and U.S. dollar denominated government bonds (nil for the nine months ended September 30, 2022).

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Net cash generated from investing activities

In the three months ended March 31, 2024, we generated $64.4 million in our investing activities compared to generating $3.0 million for the same period in 2023.

	Three months ended March 31,	
	2024	2023
	(in thousands)	
Proceeds from maturity of investment securities	$ 150,107	$ 5,330
Investment in investment securities	(83,778)	—
Build out of Amsterdam site	(1,051)	(686)

Build out of Lexington site	(1,293)	(1,656)
Net cash generated from investing activities	**$ 63,985**	**$ 2,988**

During the nine three months ended September 30, 2023 March 31, 2024, we received $106.3 million $150.1 million from the repayment of previous investments into euro and U.S. dollar denominated government bonds ($5.3 million for three months ended March 31, 2023).

During the three months ended March 31, 2024, we invested $83.8 million of our cash on hand into euro denominated government bonds (nil for nine the three months ended September 30, 2022 March 31, 2023).

We invested $2.2 million $1.1 million and $3.0 million $1.3 million, respectively, into our Amsterdam, Netherlands and Lexington, Massachusetts sites consumed during the nine three months ended September 30, 2023 March 31, 2024, compared to $8.3 million $0.7 million and $4.4 million $1.7 million for the same period in 2022. 2023.

Net cash generated from financing activities

In the nine three months ended September 30, 2023 March 31, 2024, we generated $362.7 million nil from financing activities compared to $0.8 million $0.1 million for the same period in 2022. 2023.

	Nine months ended September 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	262	844
Contingent consideration milestone payment	(7,649)	-
Net cash generated from financing activities	**$ 362,675**	**$ 844**

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

	Three months ended March 31,	
	2024	2023
	(in thousands)	
Cash flows from financing activities		
Proceeds from issuance of ordinary shares related to employee stock option and purchase plans	-	131
Net cash generated from financing activities	**$ -**	**$ 131**

During the nine three months ended September 30, 2023 March 31, 2024, we received $0.3 million nil from the exercise of options to purchase ordinary shares in relation to our 2014 Plans, compared to $0.8 million $0.1 million for the same period in 2022.

In September 2023, following the FDA's clearance of the IND application for AMT-260, we made a payment of $9.5 million to the former shareholders of uniQure France SAS to settle a portion of the contingent consideration based on contractually defined milestones, of which $7.6 million was classified as cash flows from financing activities and $1.9 million was classified as a net cash flow used in operating activities. 2023.

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;

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- 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; and

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- the costs associated with maintaining quality compliance and optimizing our manufacturing processes, including the operating costs associated with our Lexington, Massachusetts manufacturing facility;
- the financial impact of the Reorganization; and
- the costs associated with increasing the scale and capacity of our manufacturing capabilities. facility.

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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 nine three months ended September 30, 2023 March 31, 2024, 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 September 30, 2023 March 31, 2024. Based on such evaluation, our CEO and CFO concluded that as of September 30, 2023 March 31, 2024, 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.

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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 are dependent on the success of our lead product candidate in clinical development, AMT-130 for the treatment of Huntington's disease. A failure of AMT-130 in clinical development, challenges associated with its regulatory pathway, or its inability to demonstrate sufficient efficacy to warrant further clinical development could adversely affect our business.
- We have encountered and may continue to encounter future delays in and impediments to the progress of our clinical trials or failure fail to demonstrate the safety and efficacy of our product candidates.

- The price Our progress in early-stage clinical trials may not be predictive of long-term efficacy in late-stage clinical trials, and our ordinary shares has been and progress in trials for one product candidate may not be predictive of progress in the future be volatile and fluctuate substantially. trials for other product candidates.
- We may not be successful in our efforts to use our gene therapy technology platform to build a pipeline of additional product candidates or otherwise leverage our research and technology to remain competitive.
- Our future success depends on our ability to retain key executives, technical staff, and other employees and to attract, retain and motivate qualified personnel.
- Actions that we have taken to restructure our business in alignment with our strategic priorities may not be successful as effective as anticipated, may not result in cost savings to us and could disrupt our efforts to create innovative programs, platform technologies or other technologies to be competitive with others. business.
- Gene therapies are complex, expensive and difficult to manufacture. We may not be successful in our efforts to in-license could experience capacity, production or acquire product candidates technology transfer challenges 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. delays in our development or commercialization schedules or otherwise adversely affect our business.
- 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 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.
- 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 or obtain benefits by other means from 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 in order to advance the development of our product candidates, 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).
- Actions that we We had net losses in the years ended December 31, 2023 and 2022, have taken incurred significant losses in previous years and expect to restructure our business in alignment with our strategic priorities, including incur losses during the Reorganization, current and over the next several years and may not be as effective as anticipated. never achieve or maintain profitability.
- Our internal computer systems, or those The price of our collaborators or other contractors or consultants, ordinary shares has been and may fail or suffer security breaches or other errors or disruptions, which could result in a material disruption the future be volatile and fluctuate substantially.
- If we do not achieve our projected development goals in the timeframes we announce and expect, the commercialization of our product development programs, such candidates may be delayed and, as potential issues with data integrity a result, our stock price may decline.
- If we are unable to obtain and maintain patent protection for our technology and products, or loss if the scope of data. the patent protection is not sufficiently broad, our ability to successfully commercialize our products may be impaired.

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- 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.
- 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 in the conduct or completion of such trials or failing to comply with regulatory requirements.

- We rely on third parties for important aspects of our development programs. If these parties do not perform successfully or if we fail are unable to enter into or maintain an effective system key collaborations or other contractual arrangements, our business could be adversely affected.
- We face substantial competition, and others may discover, develop, or commercialize competing products before or more successfully than we do.
- Our business development strategy depends on our ability to obtain rights to key technologies through in-licenses and support the development of internal controls, we our product pipeline through out-licenses, and those efforts may not be successful.
- Our business development strategy may not produce the cash flows expected or could result in additional costs and challenges.
- We may be unable to accurately report adversely affected by unstable market and economic conditions, such as inflation, which may negatively impact our results of operations or prevent fraud or fail to meet our reporting obligations, business, financial condition and investor confidence and the market price of our ordinary shares may be materially and adversely affected. stock price.

Risks Related to the Development of Our Product Candidates

Our We are dependent on the success of our lead product candidate in clinical development, AMT-130 for the treatment of Huntington's disease. A failure of AMT-130 in clinical development, challenges associated with its regulatory pathway, or its inability to demonstrate sufficient efficacy to warrant further clinical development could adversely affect our business.

We have invested a significant portion of our development efforts and financial resources in the development of our lead clinical product candidate, AMT-130. In December 2023, we announced updated interim data from our ongoing Phase I/II clinical trials of AMT-130, including 30 months of follow-up data from the 39 patients then enrolled in our trials in the U.S. and in Europe. We also announced our plans to continue enrollment in a third cohort to investigate AMT-130 in combination with perioperative immune suppression to evaluate near-term safety, along with our plans to initiate regulatory interactions with the FDA and EMA to discuss the interim data and strategies for ongoing development of AMT-130.

There are numerous factors that could impede or otherwise negatively impact our further development of AMT-130, including, but not limited to, patient safety issues, our failure to demonstrate sufficient clinical efficacy or durability of response data to warrant further development, delays in our ability to enroll patients or challenges with regulatory authorities. Any one or combination of these factors could force us to halt or discontinue the ongoing clinical trials of AMT-130. Certain of these risk factors are heightened in the context of drug development for rare diseases like Huntington's disease in which non-traditional study designs are utilized to demonstrate efficacy and safety, including open-label studies, single arm studies, studies utilizing active comparators or natural history data, biomarkers or other forms of surrogate endpoints, which may be utilized due to the challenges inherent in designing and conducting clinical trials for severe diseases that progress slowly and that affect small patient populations. For example, in the course of our interactions with the FDA and EMA, the regulatory authorities may disagree with our interpretation of the interim safety and efficacy data we have received to date. Since AMT-130 is based on our novel gene therapy technology, we are unable predict how regulatory authorities will interpret our data or whether they will agree with our interim conclusions or trial design or whether those data may be utilized in later-stage or registrational trials. We may be required by such regulatory authorities to conduct additional randomized studies of AMT-130 beyond our existing clinical trials, which would be costly and would significantly delay the potential approval of AMT-130. We may not be able to commit sufficient capital to support additional clinical studies of AMT-130, in which case we may need to secure a development partner for AMT-130. Such partnerships may not be available, in which case we may not be able to fully fund the AMT-130 program.

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If AMT-130 fails in development as a result of any underlying problem with our technology, then we may be required to discontinue development of other product candidates that are based on the same novel therapeutic approach. We cannot be certain that AMT-130, or any of our product candidates, will be successful in clinical trials or receive regulatory approval. If we were required to, or if we chose to, discontinue development have not yet been approved for commercial sale and they might never of AMT-130 or any other future product candidates, or if any of them were to fail to receive regulatory approval or become commercially viable. We have never generated any

significant revenue achieve sufficient market acceptance, we could be prevented from product sales or significantly delayed in achieving profitability and may never our business would 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. adversely affected.

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 Drug 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. We are currently conducting Phase I/II clinical trials in the U.S. and Europe for AMT-130, our investigational gene therapy for the treatment of Huntington's disease. We are also advancing three other product candidates into clinical development – AMT-260 for the treatment of refractory mesial temporal lobe epilepsy, AMT-162 for the treatment of SOD1-ALS and AMT-191 for the treatment of Fabry disease.

We have experienced clinical setbacks in the past and may experience setbacks in the future. 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 of AMT-130 for the treatment of Huntington's disease between July and October 2022 because of due to 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 and for a variety of testing. reasons that we cannot predict with accuracy and that are out of our control. 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;
- insufficient number of patients treated with the product candidate or study period for assessing the effectiveness of the product candidate insufficient in length to assess potential clinical development;
- failures or delays in reaching a consensus agreement with regulatory agencies on study design; design, particularly with respect to our novel gene therapies for which regulatory pathways remain untested;
- failures or delays in hiring sufficient personnel with the requisite expertise to execute multiple clinical programs simultaneously;
- failures or delays in reaching agreement on acceptable terms with prospective clinical research organizations ("CROs") and clinical trial sites;
- failures or delays in patient recruiting into clinical trials or in the addition of new investigators;
- delays in receiving regulatory authorization to conduct the our clinical trials or a regulatory authority decision that the clinical trial should not proceed;
- failures or 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; trials or testing;
- 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 chosen bases for comparison as it relates to clinical efficacy, 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;

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- 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; candidates or the field of gene therapy;
- with respect to the product candidates for which we manufacture drug product in-house, 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 regulatory approval or if there are safety concerns, concerns around durability of response or other 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, safety warnings, labeling statements or safety warnings; contraindications;
- be subject to changes in the way the product is our products are 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 legal action or contraindications;
- be sued; other challenges; 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 for which we may not be able to be demonstrated to meet the regulatory authorities' standards.

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Our ability to recruit patients for our clinical trials is often heavily reliant on third parties, such as clinical trial sites. Clinical trial sites may not have the adequate infrastructure established to handle the administration of our gene therapy products, related surgeries or other means of product administration, or may have difficulty finding eligible patients to enroll into a trial.

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clinical trial, which may delay or impede our planned trials. In addition, we or any of our 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, but not limited to, 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 predictive of long-term efficacy in late-stage clinical trials, and our progress in trials for one product candidate may not be indicative predictive 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. For example, the results from early clinical trials of AMT-130, our product candidate targeting Huntington's disease, may not be predictive of the results of later-stage trials. In some instances, there can be significant variability in safety or efficacy results between different clinical trials of the same product candidate due to numerous factors, including changes in trial procedures set forth in protocols, differences in the size and type of the patient populations, changes in and adherence to the clinical trial protocols and the rate of dropout among clinical trial participants. Moreover, should there be an issue with the design of any of our clinical trials, our results may be impacted. We may not discover such a flaw until the clinical trial is at an advanced stage. Changes to product candidates, whether as a result of regulatory feedback or changes in clinical trial procedures and protocols, 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 our clinical trials, if these the results are not reproducible or if our products show diminishing activity over time, our product candidates may not receive approval from the FDA, EMA or EMA. comparable regulatory authorities. 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.

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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" interim 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 preliminary or interim analyses of data, and we may not have received or had the opportunity to evaluate all data. data at that time. As a result, the "top-line" interim 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" Interim 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" preliminary or preliminary interim data should be viewed with caution until the final data are available.

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From time to time, we also disclose interim data from our preclinical studies and clinical trials. For example, in December 2023, we announced updated interim data from our ongoing Phase I/II clinical trial of AMT-130, along with our expectation that we will present additional clinical updates with respect to AMT-130 in the future. 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 Significant 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. For example, we plan to initiate regulatory interactions in the first half of 2024 to discuss the U.S. and European data from our ongoing Phase I/II clinical trial of AMT-130 and potential strategies for ongoing development of AMT-130. These regulatory authorities may not agree with the assumptions, estimates, calculations, conclusions or analyses underlying the interim data from our ongoing clinical trial of AMT-130 or any of our future proposals regarding the ongoing development of AMT-130. Even if the data supporting such regulatory interactions are suggestive of clinical responses, the durability of response may not be sustained over time or may not be sufficient to support regulatory approval.

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" preliminary or preliminary interim 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 We are making use of exploratory biomarkers and other data that are not scientifically validated, and our reliance on these data may lead us to the PRIME

scheme by the EMA, for direct 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. resources inefficiently.

We have obtained and may are making use of experimental biological markers, or biomarkers, 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 an effort to facilitate the clinical our drug development and expedite the review to optimize our clinical trials. Biomarkers are proteins or other substances which can serve as an indicator of drugs intended to treat a serious specific cell processes 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 patient's biological response 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. drug product administration. 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 example, with respect to manufacturing or any combination medical devices, such as companion diagnostics. If our ongoing clinical trials of AMT-130, we are unable to meet these expectations, we may not be able to fully avail ourselves measuring NfL in cerebrospinal fluid ("CSF") as a potential indicator of certain advantages neurodegeneration, as well as the pharmacodynamics of these programs. mHTT in CSF and changes in total brain volume of patients treated with AMT-130.

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While we believe that these biomarkers and data may serve useful purposes for us, including in the evaluation of whether our product candidates are having their intended effects through their assumed mechanisms of action, improving patient selection and monitoring patient compliance with trial protocols, these biomarkers and data have not been scientifically validated and are considered experimental as used in our trials. If our understanding and use of biomarkers is inaccurate or flawed, or if our reliance on specific biomarkers such as NfL and mHTT is otherwise misplaced, then we may fail to realize any benefits from using these data and may also be led to invest time and financial resources inefficiently in attempting to develop inappropriate drug candidates.

We may not be successful in our efforts to use our gene therapy technology platform to build a pipeline of additional product candidates. candidates or otherwise leverage our research and technology to remain competitive.

An element of our strategy is to use our gene therapy technology platform to expand our product pipeline and to progress these our product candidates through preclinical and clinical development ourselves or together with collaborators. To date, we have only been successful in obtaining regulatory approval for one product, HEMGENIX®, our gene therapy for the treatment of hemophilia B, which was approved for commercialization by the FDA and the EMA in November 2022 and February 2023, respectively. AMT-130 is our investigational gene therapy candidate for the treatment of Huntington's disease that utilizes our proprietary, gene-silencing miQURE platform and incorporates an AAV vector carrying a miRNA specifically designed to silence the huntingtin gene and the potentially highly toxic exon 1 protein fragment, which is currently in ongoing Phase I/II studies in the U.S. and Europe. In addition to AMT-130, we are also developing other investigational gene therapies, including AMT-260 for the treatment of MTLE, AMT-162 for the treatment of SOD1 ALS and AMT-191 for the treatment of Fabry's disease. Although we currently have a pipeline of programs at various stages of development, including an approved product, 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. For example, as a result of the Reorganization, we discontinued investments in certain of our prior research and development programs, including AMT-210 for the treatment of Parkinson's disease, and certain other technology projects, prioritizing instead our early clinical-stage programs, including AMT-130, AMT-260, AMT-162 and AMT-191.

Our decisions concerning the allocation of research, development, collaboration, management, and financial resources toward particular product candidates, including the decisions stemming from our Reorganization, 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 business development strategy of obtaining depends on our ability to obtain rights to key technologies through in-licenses and support the development of our product pipeline through out-licenses, and those efforts may not be successful.

We seek to may expand our product pipeline from time to time in part by in licensing through strategic transactions that involve in-licensing the rights to key technologies, including those related to gene delivery, genes, and gene cassettes. For example, in July 2021, we acquired uniQure France (formerly Corlieve Therapeutics SAS) and its lead program, now known as AMT-260, to treat refractory MTLE. AMT-260 is being developed based on exclusive licenses to certain patents uniQure France obtained from two French research institutions that continue to collaborate with us. uniQure France also obtained an exclusive license from Regenxbio, Inc. to use AAV9 in connection with the delivery of any sequence that affects the expression of the GRIK2 gene in humans. Notwithstanding efforts to expand our product pipeline, the cost of drug development is high as is the rate of failure in the drug development process. In order to fund the development of some of our existing product candidates, we may seek to out-license some of our product candidates or technologies to other pharmaceutical or biotechnology companies or other third parties. The aim of such out-licensing would be generate non-dilutive funds in the form of up-front or milestone payments or royalties. Such decisions will be taken on a case-by-case basis, as the opportunity arises or is required.

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The future growth success of our business will depend in significant part on our business development efforts with respect to existing and future product candidates, including our ability to in-license or otherwise acquire the rights to additional product candidates or technologies, particularly through our collaborations with academic research institutions. institutions, and our ability to out-license product candidates and technologies for which collaboration with external parties forms a part of our business strategy. 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 gene therapy 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.

Similarly, there is no guarantee that we will generate product candidates that are suitable for out licensing or attractive to potential collaborators, and even if we do, there is no guarantee that we will be successful in identifying potential licensees and successfully negotiating such collaborations on agreeable terms if and when required. Any failure with respect to our business development efforts may materially affect our ability to finance our business and support the development of our product pipeline.

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.

Gene therapy remains a novel technology. Our technology utilizes vectors derived from viruses, which may be perceived as unsafe or may result in unforeseen adverse events.Public perception may be influenced by claims that gene therapy is therapies are unsafe, and gene therapy therapies may not ultimately gain the acceptance of the public or the medical community. The risk of cancer remains a concern for gene therapy, and we cannot assure guarantee that it will not occur patients treated in any of our planned or future clinical studies. studies will not develop cancer as a result of being treated with our product candidates. 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.

Public and medical community adoption of any of our gene therapies will depend on other factors, including the ease of administration in comparison to other therapeutics and the extent to which our therapies are successful in slowing disease progression if not acting as a cure for the disease. For example, the need for lengthy and 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 products prescribing treatments that involve the use of our products 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 regulation of gene therapies or negative public opinion may 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.

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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. 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) (HEMGENIX®), etranacogene dezaparvovec (AMT-061), and AMT-130. However, adverse events in our clinical trials or those conducted by other third 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.

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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 require medical devices for administration, such as AMT-130 require medical devices, such as and AMT-260, each of which requires a stereotactic, magnetic resonance imaging guided catheter, for product administration. catheter. 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. In addition, certain of our product candidates, including AMT-130 and AMT-260, may require the use of immunosuppressive agents to reduce the inflammatory responses associated with administration.

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 time and 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, or if there is reluctance to administer immunosuppressive agents that are outside of the standard of care to treat immune responses from the administration of our therapies, 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 facilities are 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.

With the exception of AMT-260 and AMT-162, we produce our gene therapies at our Lexington Facility using a proprietary baculovirus expression vector system. Our manufacturing facility in Lexington MA Facility, where we manufacture HEMGENIX®, is subject to ongoing regulation and periodic inspection by the FDA, EU member state, and other regulatory bodies to ensure compliance with current cGMP and other 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.

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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. 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; or
- 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, or if we are unable to manufacture sufficient supply of HEMGENIX® consistent with our manufacturing and supply obligations to CSL Behring, our development program programs and eventual commercial prospects will be harmed. If we cannot produce an adequate amount of our drug substance and 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 to us on favorable terms. terms or at all. 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.

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

The insect-cell based Our proprietary manufacturing process we use leveraging insect cells and baculoviruses to produce our products and product candidates to AAV-based gene therapies is highly complex and in the normal course is regularly subject to variation or production difficulties. Issues with any of our manufacturing processes, even minor deviations from the normal process, our standard processes, 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 as necessary and on our desired timelines to meet the demands of our clinical product pipeline, which may result in delays in regulatory approvals, inability to produce sufficient amounts of clinical or commercial product, or otherwise adversely affect our ability to manufacture sufficient amounts of our products. business.

Many factors Factors common to the manufacturing of process associated with most biologics and drugs could also cause production interruptions for us, including, without limitation, raw materials shortages and other supply chain challenges, raw material failures, limited control over pricing of raw materials, growth media failures, equipment malfunctions, costs associated with servicing real property lease and

other contractual obligations, facility contamination, labor problems, natural disasters, disruption in utility services, public health crises, terrorist activities, war or cases of force majeure and acts of God (including the effects of the COVID-19 pandemic) that are beyond our control. We also may encounter problems in hiring and retaining the experienced and specialized personnel needed to operate our manufacturing process, facilities, processes and testing, which could result in delays in our production or difficulties in maintaining compliance with applicable regulatory requirements.

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We manufacture HEMGENIX® at our Lexington Facility which is optimized to meet our commercial manufacturing and supply obligations pursuant to the CSL Behring collaboration. This optimization and dedicated capacity for HEMGENIX® could limit our ability to manufacture other product candidates or components thereof to support our development programs or those of third parties. The manufacturing of HEMGENIX® pursuant to our obligations under the CSL Behring Agreement is expensive and requires the dedication of significant company resources. In September 2022, CSL Behring notified us of its intent to transfer manufacturing technology in the coming years related to HEMGENIX® to a third-party contract manufacturer to be designated by CSL Behring in the future. Until CSL Behring identifies and designates a new manufacturer capable of supporting the commercial requirements of HEMGENIX®, we will continue to incur significant costs associated with our manufacturing and supply obligations. Following such transfer, we may experience challenges in adapting our Lexington Facility to meet the manufacturing and supply needs for products other than HEMGENIX® as a result of excess capacity or our ability to adapt to new processes, among other challenges. Any problems in or limitations with respect to our manufacturing processes or facilities, including our existing commercial supply and manufacturing obligations to CSL Behring, could make us a less attractive collaborator for academic research institutions and other parties, which could limit our access to additional attractive development programs or sources of capital, result in delays in our clinical development or marketing schedules and materially harm our business.

We currently rely and expect to continue to rely on third parties to conduct product manufacturing for certain of our product candidates, and these third parties may not perform satisfactorily.

We currently rely, and expect to continue to rely, on third parties for the production of some of our preclinical study and planned clinical trial materials and, therefore, we can control only certain aspects of their activities. The facilities used by us and our contract manufacturers to manufacture certain of our product candidates must be reviewed by the FDA pursuant to inspections that will be conducted after we submit a BLA to the FDA. We do not control the manufacturing process of, and are completely dependent on, our contract manufacturing partners for compliance with the cGMP for the manufacture of our products. products and product candidates that are not manufactured in house. If we or our contract manufacturers cannot successfully manufacture material that conforms to our specifications and the strict regulatory requirements of the FDA or others, other regulatory bodies, we will not be able to obtain and/or maintain regulatory approval for our products as manufactured at their manufacturing facilities. by third parties. In addition, we have no control over the ability of our contract manufacturers to maintain adequate quality control, quality assurance and qualified personnel. If the FDA or a comparable foreign regulatory authority does not approve these facilities for the manufacture of our product candidates or if it withdraws any such approval in the future, we may need to find alternative manufacturing facilities, third-party manufacturers, which may not be available and which would significantly impact our ability to develop, obtain regulatory approval for or market our product candidates, if approved.

Our use of viruses, chemicals and other potentially 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) 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 we are subject to 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.

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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 for each program, 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 our product candidate. candidates.

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. products, which may adversely affect the approval prospects for our product candidates.

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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 will likely must comply be applicable to gain regulatory approval of any of our product candidates prior to our obtaining regulatory approval in the U.S. or EU, respectively. the EU. 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. Experiences with existing gene therapies, including any emergent adverse effects, could also impact how the FDA and the EMA view our products and product candidates, making it harder to obtain or maintain regulatory approvals.

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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.

We may use certain specialized pathways to develop our product candidates or to seek regulatory approval. We may not qualify for these pathways, or such pathways may not ultimately speed the time to approval or result in product candidate 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 accelerate the FDA marketing application review timeframe for drug products that treat a serious condition and that, 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.

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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., the 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.

Biologics studied for their safety and effectiveness in treating serious or life-threatening illnesses and that provide meaningful therapeutic benefit over existing treatments may receive accelerated approval by the FDA, meaning the agency may approve the product candidate based upon a surrogate endpoint that is reasonably likely to predict clinical benefit, or on a clinical endpoint that is reasonably likely to predict an effect on irreversible morbidity or mortality or other clinical benefit. Even if we do qualify for accelerated approval, we may be unsuccessful in meeting post-marketing compliance requirements, or fail to conduct required post-approval studies, or to confirm a clinical benefit during post-marketing studies, which could result in the FDA withdrawing our product from the market. In recent years, the accelerated approval pathway has come under significant FDA and public scrutiny. Accordingly, it is uncertain whether the FDA may be more conservative in granting accelerated approval or, if granted, more apt to withdraw approval if clinical benefit is not confirmed. There is no guarantee that regulatory interactions with FDA or comparable foreign authorities will result in our ability to avail ourselves of any specialized approval pathways for our product candidates.

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, including AMT-130 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.

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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.

Table Our focus on developing gene therapies makes it difficult to determine the availability and utility of Contents

Additionally, regulatory the orphan drug regime to our product candidates. 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 gene therapies 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.

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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 on 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.

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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.

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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, including us, 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. If we or any of our contractors are unable to comply with the requirements that are applicable to drug manufacturers, we or they may be subject to regulatory enforcement, or may need to conduct a recall or take other corrective actions, which could result in material harm to us or our 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 the achievement 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;

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- 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.

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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. diseases and may change. 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. reimbursement, among other factors.

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. For example, the addressable markets for certain of our 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 might not be eligible for administration of a gene therapy that includes this particular capsid. Moreover, neutralizing antibodies may be developed by a patient following administration of the product, which may render the patient ineligible for subsequent dosing. The use of such data to support addressable market estimates involves risks and uncertainties and is subject to change based on various factors. Our estimates may prove to be incorrect and new studies and information 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.

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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 associated with genetic testing 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 patient's underlying 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;

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- 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 current and future 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, like us, currently market and sell products or are pursuing the development of products for the treatment of many rare diseases.

Table of the disease indications for which we are developing our product candidates. Contents

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 (AskBio), Amicus Therapeutics, and 4D Molecular Therapeutics. Therapeutics, Sanofi, Idorsia, Amicus, Spark, Takeda, Chiesi, CANbridge, Abeona, Annexon, Vico, Alexion (AZ), Neurona, Combigene, NeuExcell, EpiBlok, Biogen, ionis, Eisai and Lexeo.

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.

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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. Moreover, actions taken in connection with the Reorganization to streamline our product portfolio may hamper our ability to remain competitive. 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 in the conduct and 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.

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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.

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Our reliance on these third parties for development activities will reduce reduces 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.

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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 we enter into 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;

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- 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.

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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, may be open to multiple interpretations 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 are 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, or have otherwise given up the right, to control the preparation, filing and prosecution of patent applications, or to maintain the patents, covering technology that we own or 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 which may materially impact any revenue that may be due to us in connection with such patents. 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.

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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. agreement or may otherwise result in reputational damage to our business. 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.

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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. Additionally, the patent prosecution process is expensive, time-consuming, and uncertain, and in certain instances we have chosen, and in the future we may choose, not to file and prosecute all necessary or desirable patent applications. For example, our defense of certain patent cases in each of Canada, the United Kingdom, the Netherlands and the U.S. pertaining to licensed rights of etranacogene dezaparvovec was assumed by CSL Behring on October 11, 2023. These oppositions and future patent oppositions may result in loss of scope of some claims or the entire patent and, with respect to our rights under the CSL Agreement, could affect CSL's successful commercialization of HEMGENIX® and, in turn, could negatively impact our financial position. Additionally, 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 or the ability of our licensees 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.

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.

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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.

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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' 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 or patent applications 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 positions 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.

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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.

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The occurrence of 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.

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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.

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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 many 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 net losses in the nine months years ended September 30, 2023 December 31, 2023 and year ended December 31, 2022, 2022, have 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 $235.3 million $65.6 million in the nine three months ended September 30, 2023 March 31, 2024, and a net loss of $126.8 million $308.5 million in the full year 2022. ended December 31, 2023. We incurred a gain of $329.6 million in year ended December 31, 2021; however, such gain was primarily attributable to one-time license revenue from CSL Behring. We have incurred significant losses in the years prior to 2021. As of September 30, 2023 March 31, 2024, we had an accumulated deficit of $817.2 million $956.0 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 2024 and until into the second quarter of 2027 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 we will continue to incur net losses for the foreseeable future as we:

- continue to fund AMT-130 in its ongoing clinical trials and advance our other product candidates into clinical development;
- incur the costs associated with the manufacturing of preclinical, clinical and commercial supplies of our product candidates;
- seek regulatory approvals for any product candidates that successfully complete clinical trials;
- maintain, expand and protect our intellectual property portfolio;
- hire additional personnel to support our business;
- enhance our operational, financial and management information systems and personnel; and
- incur legal, accounting and other expenses operating as a public company.

As While we expect that, as a result of the Reorganization, we expect to will realize some cost savings and reduce our operating expenses, until such time that we decide to advance our gene therapy product candidates into late stage clinical development. We may never succeed in these activities materially reducing our operating expenses and, even if we do, may never generate revenues that are sufficient to achieve or sustain profitability. Even if we do achieve profitability, we may not be able to sustain or increase profitability on a quarterly or annual basis. Our failure to become and remain profitable would depress the value of our company and could impair our ability to raise capital, expand our business, maintain our research and development efforts, diversify our product offerings, or even continue our operations.

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We will likely need to raise additional funding in order to advance the development of our product candidates, 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 order to fund the development of our product pipeline and support 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 additional 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. Our ability to obtain additional equity financing may be limited by our shareholders' willingness to approve the issuance of additional share capital. 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 further 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 September 30, 2023 December 31, 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 might not be able to finance our operations into the second quarter of 2027 from our existing cash, cash equivalents, and cash resources if we are not able to refinance the 2023 Amended Facility prior to the January 2027 maturity date. 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 2023 Amended Facility 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.

Our 2023 Amended Facility bears a variable interest rate with a fixed floor. The U.S. Federal Reserve has raised, and may in the future further raise, interest rates to combat the effects of recent high inflation. An increase in interest rates by the Federal Reserve has and could in the future cause the prime rate to increase, which has and could in the future increase our debt service obligations. Significant increases in such obligations could have a negative impact on our financial position or operating results, including cash available for servicing our indebtedness, or result in increased borrowing costs in the future

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

In July 2021, we acquired uniQure France and its lead program, now known as AMT-260, targeting refractory MTLE, and may, from time to time, enter into strategic transactions consistent with our business development objectives. Any acquisition or strategic 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 necessary to align our business practices and operations with that of the acquired company. Moreover, we cannot be sure that the anticipated or intended benefits of any acquisition or strategic transaction would be realized in a timely manner, if at all.

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.

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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 an alleged or actual 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, international, 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 EU General Data Protection Regulation ("GDPR") that took effect in May 2018. The UK has, following its exit from the EU, substantially adopted the EU General Data Protection Regulation into its domestic law through the UK General Data Protection Regulation (collectively with the EU General Data Protection Regulation, and related EU and UK e-Privacy laws, the "GDPR"). The GDPR, together with the national legislation of the UK (including the Data Protection Act 2018) and EU member states governing the processing of personal data,

imposes impose strict obligations and restrictions on the ability to collect, use, analyze and transfer personal data, information, including health data from clinical trials and adverse event reporting. In particular, these

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GDPR obligations and restrictions concern applicable to us may include, in many circumstances, obtaining the (opt-in) consent of the individuals to whom the personal data relates, the information provided relates; providing GDPR-prescribed data processing notices to the individuals, individuals; complying with restrictions regarding the transfer of personal data out of the EU or the UK (as applicable) (including to the US); implementing and maintaining data protection policies and procedures; restrictions regarding the use of certain innovative technologies; providing data security breach notifications to supervisory authorities and affected individuals under tight timescales; and implementing 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 measures. Supervisory authorities from in the different EU member states and the UK may interpret the GDPR and national laws differently and impose additional requirements, which add to the complexity of processing personal data in the EU. requirements. Guidance on implementation and compliance practices are often updated or otherwise revised. All of this adds to the complexity of processing personal information and remaining compliant with the GDPR.

The GDPR allows EU and UK supervisory authorities to impose penalties for non-compliance of up to the greater of EUR 20.0 million and 4% of annual worldwide gross revenue of the corporate group in question. (There are similar caps in GBP under the UK GDPR.). Supervisory authorities in the EU and UK may potentially levy such fines directly upon on the non-compliant entity and/or on the parent company of the non-compliant entity. Supervisory authorities also possess other wide-ranging powers, including conducting unannounced inspections of our facilities and system (so-called “dawn raids”), and issuing “stop processing” orders to us. Separate from regulatory enforcement actions, individuals may bring private actions (including potentially group or representative actions) against us. There is no statutory cap in the GDPR on the amount of compensation or the damages which individuals may recover.

Overall, the significant costs of GDPR compliance, with risk of regulatory enforcement actions and private litigation 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 or the procedures used to administer them to patients 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 a 1% payment adjustment was implemented from April 1 – June 30, 2022, and a 2% payment adjustment took effect through 2031 unless additional congressional action is taken. beginning July 1, 2022. 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 117 118th 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 outside of the U.S. and Europe 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 jurisdictions we must comply with numerous and varying regulatory requirements of such countries jurisdictions 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.

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

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

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. We may need to devote significant resources to protect against security breaches or to address problems caused by a cyber-attack or security breach. 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.

See Part I, Item 1C, *Cybersecurity*, in our Annual Report for more information regarding our cybersecurity risk management, strategy and governance.

Climate change as well as corporate responsibility initiatives, including environmental, social and governance and sustainability initiatives (ESG) matters, 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 impose additional costs on our business financial condition, and results of operations. expose us to new risks.

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 the impact of 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, increases in taxes, transportation cost, costs and utility increases. utilities, among other expenses. Moreover, natural disasters and extreme weather conditions may impact the productivity of our facilities, the ability of the patients in our clinical trials to maintain compliance with trial protocols or access clinical trial sites, the operation of our supply chain, or consumer buying patterns. Any The occurrence of any of these risks events could have a material adverse effect on our business.

Climate change, environmental, social and governance ESG 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 continue to attract political and social attention to these issues has have resulted in both existing and pending international agreements and national, regional, and local legislation, regulatory measures, reporting obligations and policy changes. Also, there There is increasing societal pressure in some of the areas where countries in which we operate to limit greenhouse gas emissions as well as other global initiatives. initiatives focused on climate change. 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 require the that we dedicate additional resources toward compliance with these measures and result in substantial capital expenditures.

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Furthermore, increasing attention to climate change, sustainability and environmental, social and governance ("ESG") on ESG matters 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.

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In addition, organizations that provide information to investors on corporate governance and related matters have developed ratings processes for evaluating companies and investment funds based on their approach to ESG matters. and sustainability metrics. 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. In addition, investors, particularly institutional investors, use these scores to benchmark companies against their peers and if a company is perceived as lagging, take actions to hold these companies and their boards of directors accountable. Board diversity is an ESG topic that is, in particular,

receiving heightened attention by investors, stockholders, lawmakers and listing exchanges. Certain states have passed laws requiring companies to meet certain gender and ethnic diversity requirements on their boards of directors. We may face reputational damage in the event our corporate responsibility initiatives or objectives, do not meet the standards set by our investors, stockholders, lawmakers, listing exchanges or other constituencies, or if we are unable to achieve an acceptable ESG or sustainability rating from third-party rating services.

Any The effects of climate change or 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 Our 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.

Actions that we have taken to restructure our business in alignment with our strategic priorities may not be as effective as anticipated, may not result in cost savings to us and could disrupt our business.

In October 2023, we commenced the Reorganization to reprioritize our portfolio of development candidates, conserve financial resources and better align our workforce with current business needs. We may encounter challenges in the execution of these efforts, and these challenges could impact our financial results.

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Although we believe that these actions will reduce operating costs, we cannot guarantee that the Reorganization will achieve or sustain the targeted benefits, or that the benefits, even if achieved, will be adequate to meet our long-term expectations. As a result of the Reorganization, we will incur additional costs in the near term, including cash expenditures for employee transition, notice period and severance payments, employee benefits, and related facilitation costs. Additional risks associated with the continuing impact of the Reorganization include employee attrition beyond our intended reduction in force and adverse effects on employee morale (which may also be further exacerbated by actual or perceived declining value of equity awards), diversion of management attention, adverse effects to our

reputation as an employer (which could make it more difficult for us to hire and retain new employees in the future), potential understaffing and potential failure or delays to meet development targets due to the loss of qualified employees. employees or other operational challenges. If we do not realize the expected benefits of our restructuring efforts on a timely basis or at all, our business, results of operations and financial condition could be adversely affected.

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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 November 2, 2023, May 2, 2024 the sale price of our ordinary shares ranged from a high of $82.49 to a low of $4.72. $4.39. The closing price on November 2, 2023 May 2, 2024, was $5.72 $4.68 per ordinary share.

In recent years, the stock market in general and the market for shares of smaller biopharmaceutical companies in particular have experienced significant price and volume fluctuations that have often been unrelated or disproportionate to changes in the operating performance of the companies whose stock is experiencing those price and volume fluctuations. 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;
- changes to our business, including pipeline reprioritizations and restructurings;
- 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;
- general economic, industry and market conditions; and
- the other factors described in this “Risk Factors” section.

Following periods of such volatility in the market price of a company’s securities, securities class action litigation has often been brought against that company. Because of the potential volatility of our stock price, we may become the target of securities litigation in the future. In addition, notwithstanding protective provisions in our articles of association and available to us under Dutch corporate law, market volatility may lead to increased shareholder activism if we experience a market valuation that activist investors believe is not reflective of the intrinsic value of our ordinary shares. Activist campaigns that contest or conflict with our strategic direction or seek changes in the composition of our board of directors could have an adverse effect on our operating results and financial condition. Securities litigation or shareholder activism could result in substantial costs and divert management’s attention and resources from our business. 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;
- changes to our business, including pipeline reprioritizations and restructurings;
- 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;
- general economic, industry and market conditions; and
- the other factors described in this "Risk Factors" section.

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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 36.6% 26.5% of our issued shares (including such shares to be issued in relation to exercisable options to purchase ordinary shares) as of September 30, 2023 March 31, 2024. 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.

Under Dutch law, various protective measures are possible and permissible within the boundaries set by Dutch statutory and case law. 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:

- the staggered three-year terms of our directors; non-executive directors as a result of which only approximately one-third of our non-executive directors may be subject to election or re-election in any one year;
- a provision that our directors may only be removed dismissed or suspected at a general meeting of shareholders by a two-thirds majority of votes cast representing more than half of the issued share capital our outstanding ordinary shares;
- a provision that our executive directors may only be appointed upon binding nomination of the Company; non-executive directors, which can only be overruled by the general meeting of shareholders with a two-thirds majority of votes cast representing at least 50% of our outstanding ordinary shares; 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.

Table Moreover, according to Dutch corporate law, our board can invoke a cooling-off period of Contents up to 250 days in the event of an unsolicited takeover bid or certain shareholder activism. During a cooling-off period, our general meeting of shareholders would not be able to dismiss, suspend or appoint directors (or amend the provisions in our articles of association dealing with those matters) except at the proposal of 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. Holders65

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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 passive foreign investment company ("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. Based on our average value of our gross assets, our cash and cash equivalents as well as the price of our ordinary shares, we expect to be classified as a PFIC for U.S. federal income tax for 2023. 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 organized and existing under the laws of the Netherlands. Some of the members of our board and senior management reside outside the U.S. In addition, a significant portion of our assets are located 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.

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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.

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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 We are a U.S. domestic filer for SEC purposes, public company (*naamloze vennootschap*) organized under the laws of the Netherlands and 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, it is possible that some of these parties will have interests that are different from, or in addition to, your interests as a shareholder, and 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.

In addition, in accordance with our articles of association, approval of our shareholders is required before our board of directors can authorize the issuance of our ordinary shares in an equity financing. Our shareholders' reluctance to approve such further issuances of ordinary shares could adversely affect our ability to raise capital and fund development programs and continued operations. There can be no assurance that Dutch law will not change in the future or that it will serve to protect investors in a similar fashion afforded under corporate law principles in the U.S., which could adversely affect the rights of investors.

We may be adversely affected by unstable market and economic conditions, such as inflation, which may negatively impact our business, financial condition and stock price.

Market conditions such as inflation, volatile energy costs, geopolitical issues, war, unstable global credit markets and financial conditions could lead to periods of significant economic instability, diminished liquidity and credit availability, diminished expectations for the global economy and expectations of slower global economic growth going forward. Our business and operations may be adversely affected by such instability, including any such inflationary fluctuations, economic downturns, volatile business environments and continued unstable or unpredictable economic and market conditions. Inflation in particular 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 across our business. 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.

Any such volatility and disruptions may have adverse consequences on us or the third parties on whom we rely. If economic and market conditions deteriorate or do not improve, it may make any future financing efforts more difficult to complete, more costly and more dilutive to our shareholders. Additionally, due to our volatile industry and industry-wide declining stock values, investors may seek to pursue non-biotech investments with steadier returns. Failure to secure any necessary financing in a timely manner or on favorable terms could have a material adverse effect on our operations, financial condition or stock price or could require us to delay or abandon development or commercialization plans.

If securities or industry analysts cease to publish or publish inaccurate or unfavorable research about our business, our share price and trading volume could decline.

The trading market for our common shares depends in part on the research and reports that securities or industry analysts publish about us or our business. If one or more of the analysts who cover us downgrades our common shares or publishes inaccurate or unfavorable research about our business, our share price may decline. If one or more of these analysts ceases coverage of our company or fails to publish reports on us regularly, demand for our shares could decrease, which might cause our share price and trading volume to decline.

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If we do not achieve our projected development and financial 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.

We estimate the timing of the accomplishment of various scientific, clinical, regulatory, and other product development goals, along with financial and other business-related milestones. From time to time, we publicly announce the expected timing of some of these milestones along with guidance as to our cash runway. These milestones may include the commencement or completion of scientific studies, clinical trials, the submission of regulatory filings and interactions with regulatory authorities, and approval timelines for commercial sales. All these milestones are based on a variety of assumptions that may prove to be untrue. The timing of our actual achievement 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 development and commercialization of our products may be delayed, our business could suffer reputational harm and, as a result, our stock price may decline

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Item 2. Unregistered Sales of Equity Securities, Use of Proceeds, and Issuer Purchases of Equity Securities

None.

Item 3. Defaults Upon Senior Securities

None.

Item 4. Mine Safety Disclosures

Not applicable.

Item 5. Other Information

During the three months ended September 30, 2023 March 31, 2024, 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.

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EXHIBIT INDEX

10.1* t

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 September 30, 2023 March 31, 2024, filed with the Securities and Exchange Commission on November 7, 2023 May 7, 2024, 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 September 30, 2023 March 31, 2024, filed with the Securities and Exchange Commission on November 7, 2023 May 7, 2024, is formatted in Inline Extensible Business Reporting Language ("iXBRL")

* Filed herewith.

± Furnished herewith.

t Indicates a management contract or compensatory plan or arrangement.

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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 November 7, 2023 Date: May 7, 2024

Exhibit 10.1

October 4, 2023

Dear Ricardo,

The purpose of this letter agreement ("Letter Agreement") is to address the termination of your Employment Agreement for Good Reason and our mutual agreement to forego the Good Reason Process set forth in Section 19(f) thereof. Capitalized terms used but not defined in this Letter Agreement shall have the meaning set forth in the employment agreement between you and the Company made and entered into as of September 14, 2020 (the "Employment Agreement").

Your termination of employment for Good Reason will be effective on October 4, 2023 (the "Termination Date"). Provided that you timely execute and do not revoke the General Release of Claims attached as Exhibit A and otherwise comply with the requirements set forth in the Employment Agreement, you will receive the severance benefits set forth in Sections 19(h)(i)(a), (ii), and (iii) of the Employment Agreement as soon as administratively practicable (but no later than forty-five (45) calendar days) following the date that you sign and deliver the General Release of Claims to the Company and any applicable revocation period has expired, in accordance with Section 20 of the Employment Agreement.

In addition, the Company would like to retain your services as a consultant from the Termination Date until December 31, 2023 pursuant to the terms and conditions of the Consulting Agreement attached as Exhibit B (the "Consulting Agreement"). If the terms of the Consulting Agreement are acceptable to you, please execute and return the Consulting Agreement to me.

Thank you for valuable contributions to the Company.

By: _____

Name: Matt Kapusta

Title: Chief Executive Officer

Agreed to and Acknowledged by:

_____

Ricardo E. Dolmetsch Ph.D.

_____

Date

uniQure Inc.
113 Hartwell Avenue
Lexington, MA 02421

uniQure Biopharma BV
Paasheuvelweg 25A
P.O. Box 22506
1100 DA Amsterdam

Exhibit A

GENERAL RELEASE OF CLAIMS

In exchange for the promises and benefits set forth in Sections 19(h)(i)(a), (ii), and (iii) of the Employment Agreement between uniQure, Inc. (the "Company") and Ricardo E. Dolmetsch, made and entered into as of September 14, 2020 (the "Employment Agreement"), I**, Ricardo E. Dolmetsch** on behalf of myself, my heirs, executors and assigns, hereby acknowledge, understand and agree as follows:

1. On behalf of myself and my family, heirs, executors, administrators, personal representatives, agents, employees, assigns, legal representatives, accountants, affiliates and for any partnerships, corporations, sole proprietorships, or other entities owned or controlled by me, I fully release, acquit, and forever discharge the Company, its past, present and future officers, directors, shareholders, agents, representatives, insurers, employees, attorneys, subsidiaries, affiliated corporations, parents, and assigns (collectively, the "Releasees"), in their corporate and personal capacities, from any and all charges, actions, causes of action, claims, grievances, damages, obligations, suits, agreements, costs, expenses, attorneys' fees, or any other liability of any kind whatsoever, suspected or unsuspected, known or unknown, which at any time prior to my signing this General Release of Claims ("General Release") have or could have arisen out of my employment with or services performed for Releasees and/or termination of my employment with or termination of my services performed for Releasees (collectively, "Claims"), including:

a. Claims arising under Title VII of the Civil Rights Act of 1964 (as amended); the Civil Rights Acts of 1866 and 1991; the Americans With Disabilities Act; the Family and Medical Leave Act; the Employee Retirement Income Security Act; the Occupational Health and Safety Act; the Sarbanes-Oxley Act; the Massachusetts Law Against Discrimination (M.G.L. c. 151B, et seq., and/or any other laws of the Commonwealth of Massachusetts related to employment or the separation from employment);

b. Claims for age discrimination arising under the Age Discrimination in Employment Act of 1967 (as amended) ("ADEA") and the Older Workers Benefits Protection Act, except ADEA claims that may arise after the execution of this General Release;

c. Claims arising out of any other federal, state, local or municipal statute, law, constitution, ordinance or regulation; and/or

d. Any other employment related claim whatsoever, whether in contract, tort or any other legal theory, arising out of or relating to my employment with the Company and/or my separation of employment from the Releasees.

e. Excluded from this General Release are any claims that cannot be released or waived by law. This includes, but is not limited to, my right to file a charge with or participate in an investigation conducted by certain government agencies, such as the EEOC. I acknowledge and agree, however, that, to the extent permitted by law, I am releasing and waiving my right to any monetary recovery should any government agency pursue any claims on my behalf that arose prior to the effective date of this General Release.

f. I waive all rights to re-employment with the Releasees. If I do apply for employment with the Releasees, the Releasees and I agree that the Releasees need not employ me, and that if the Releasees decline to employ me for any reason, they shall not be liable to me for any cause of action or damages whatsoever.

2. Release of Other Claims. I fully release, acquit, and forever discharge the Releasees from any and all other charges, actions, causes of action, claims, grievances, damages, obligations, suits, agreements, costs, expenses, attorneys' fees or any other liability of any kind

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whatsoever related to my employment with the Company, the Employment Agreement, termination of my employment with the Company, or the business of the Company of which I have knowledge as of the time I sign this General Release.

3. I further acknowledge that I have received payment, salary and wages in full for all services rendered in conjunction with my employment with the Company, including payment for all wages, bonuses, and accrued, unused paid time off, and that no other compensation is owed to me except as provided herein (including, without limitation, the severance benefits set forth in Sections 19(h)(i)(a), (ii), and (iii) of the Employment Agreement) or in the Consulting Agreement. I specifically understand that this General Release includes, without limitation, a release of claims for alleged failure to pay earned wages, failure to pay overtime, failure to pay earned commissions, failure to timely pay wages, failure to pay accrued vacation or holiday pay, failure to furnish appropriate pay stubs, improper wage deductions, failure to provide proper check-cashing facilities or other compensation or payment including any claim for treble damages, attorneys' fees and costs pursuant to the Massachusetts Wage Act and State Overtime Law M.G.L. c. 149, §§148, 150 *et seq.* and M.G.L. c. 151, §1A *et seq*.

and I further acknowledge that I am unaware of any facts that would support a claim against the Released Parties for violation of the Fair Labor Standards Act or the Massachusetts Wage Act.

4. Notwithstanding anything to the contrary herein, nothing in this General Release shall be deemed to release any of the Releasees for: (i) any claim for any of the Accrued Benefits as defined by the Employment Agreement or (ii) any rights to indemnification or coverage under a directors and officers liability insurance policy.

5. Restrictive Covenants. I acknowledge and agree that all of my obligations under the restrictive covenants in my Confidentiality, Inventions, and Restrictive Covenants Agreement (the "CIRCA") remain in full force and effect and shall survive the termination of my employment with the Releasees and the execution of this General Release. I further acknowledge that I shall be bound by the non-competition provision set forth in Section 5(c) of the CIRCA for a period of 12 months following my employment termination date.

6. Consultation with Attorney. The Company hereby advises and encourages me to consult with an attorney prior to executing this General Release. I acknowledge that if I have executed this General Release without consulting an attorney, I have done so knowingly and voluntarily.

7. Period for Review. I acknowledge that I have been given at least 21 days from the date I first received this General Release during which to consider signing it.

8. Revocation of General Release. I acknowledge and agree that I have the right to revoke my acceptance of this General Release if I notify the Releasees in writing within 7 business days following the date I sign it. Any revocation, to be effective, must be in writing, signed by me, and either: a) postmarked within 7 business days of the date I signed it and addressed to the then current address of the Company's headquarters (to the attention of the Chief Legal Officer); or b) hand delivered within 7 business days of execution of this General Release to the Company's Chief Legal Officer. This General Release will become effective on the 8th day after I sign it (the "Effective Date"); provided that I have not timely revoked it.

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Exhibit A

I ACKNOWLEDGE AND AGREE THAT I HAVE BEEN ADVISED THAT THE GENERAL RELEASE IS A LEGAL DOCUMENT, AND I HAVE BEEN ADVISED TO CONSULT WITH AN ATTORNEY CONCERNING THIS GENERAL RELEASE. I ACKNOWLEDGE AND AGREE THAT I HAVE CAREFULLY READ AND FULLY UNDERSTAND ALL PROVISIONS OF THIS GENERAL RELEASE AND I AM VOLUNTARILY AND KNOWINGLY SIGNING IT.

IN WITNESS WHEREOF, I have duly executed this Agreement under seal as of the ___ day of _____, 2023.

By: _____
Ricardo E. Dolmetsch Ph.D.

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Exhibit B

CONSULTING AGREEMENT

THIS CONSULTING AGREEMENT (the "Agreement"), made effective October 4, 2023 (the "Effective Date"), is entered into by uniQure, Inc., a Delaware corporation with its principal place of business at 113 Hartwell Ave., Lexington, Massachusetts, 02421 (the "Company"), and Ricardo Dolmetsch (the "Consultant") (together, the "Parties").

INTRODUCTION

WHEREAS, the Consultant served as the President and Chief Scientific Officer of the Company, until October 4, 2023 (the "Separation Date");

WHEREAS, the Company and the Consultant desire to establish the terms and conditions under which the Consultant will provide transition services to the Company.

NOW THEREFORE, in consideration of the mutual covenants and promises contained herein and other good and valuable consideration, the receipt and sufficiency of which is hereby acknowledged by the Parties hereto, the Parties agree as follows:

1. Services and Work Product. As of the Effective Date, the Consultant agrees to perform such advisory and other services for the Company, as may reasonably be requested from time to time by the Company and mutually agreed by the Parties (the "Services"). Consultant shall provide no more than five (5) hours of Services per week to the Company. The Consultant shall perform the Services under the direction of the Company's CSO or CMO. All work product, research, deliverables, memoranda, data, notes, and all other documents created by the Consultant that arise out of the Services during the Term, whether or not copyrightable or privileged ("Work Product"), shall be the sole property of the Company.

2. Term. This Agreement shall become effective on the Effective Date and shall expire on December 31, 2023, unless sooner terminated in accordance with the provisions of Section 3 (the "Term"). As of the Separation Date, the Consultant's roles and responsibilities as an employee of the Company terminated, including any and all roles as an officer, or director of the Company and each of the Company's affiliates, and his Employment Agreement effective September 14, 2020 (the "Employment Agreement") terminated, except as otherwise provided in this Agreement.

3. Termination. The Company may, without prejudice to any right or remedy it may have due to any failure of the Consultant to perform his obligations under this Agreement, terminate this Agreement without prior notice upon the Company's determination that: (a) the Consultant has not performed the Services provided for hereunder to its reasonable satisfaction, as determined by the Company in its reasonable discretion, (b) the Consultant has engaged in any misconduct that has the effect, or potential effect, of causing harm to the Company (monetarily, reputationally or otherwise), (c) the Consultant has breached the principles, laws, and standards set forth in Section 8(a) of this Agreement, or (d) a conflict has arisen pursuant to Section 8(c) of this Agreement which cannot be resolved. In addition, in the event that the Consultant does not execute the General Release of Claims between the Consultant and the Company previously provided to the Consultant (the "Release") by the 21st day following the Consultant's receipt of the Release (the "Release Deadline") or the Consultant timely revokes the Release, then this

Agreement shall terminate on the earlier of the day immediately following the Release Deadline or the date of such revocation. The Consultant may terminate this Agreement for any reason at any time upon thirty (30) days' written notice to the Company. In the event of termination of this Agreement for any reason, the Consultant shall be entitled to payment hereunder for Services actually rendered and for expenses actually paid or incurred prior to the effective date of termination. Such payments shall constitute full and final settlement of all claims of the Consultant pursuant to this Agreement against the Company.

4. Compensation.

(a) Consulting Fees. During the Term, the Company shall pay to the Consultant as consideration for the Services a consulting fee of $10,000 per month (pro-rated for any partial month), which the Company shall pay in equal weekly installments or pursuant to another agreed upon payment schedule and less any applicable withholding. Payment shall be made by bank transfer to the Consultant. The Parties represent and warrant that, to the best of their knowledge, the compensation constitutes fair market value for the Services.

(b) Treatment of Existing Equity Awards. This Agreement is intended to be effective immediately upon termination of the Consultant's employment such that the Consultant's contractual relationship with the Company is continuous, and the Consultant continues to vest in awards (the "Equity Awards") granted to the Consultant pursuant to the 2014 Share Incentive Plan of uniQure N.V., provided, however, that the vesting of the Equity Awards shall cease as of the last day of the Term. For the avoidance of doubt, (i) any Equity Awards or portions thereof which have not vested as of the last day of the Term shall immediately terminate for no consideration and the Consultant will have no further rights with respect thereto, and (ii) the Consultant shall cease to be an Eligible Participant (as defined in the award agreements evidencing the Equity Awards) on the last day of the Term.

(c) Reimbursement of Expenses. The Company shall reimburse the Consultant for all reasonable and necessary expenses incurred or paid by the Consultant and approved by the Company in connection with, or related to, the performance of his services pursuant to this Agreement, subject to the receipt of copies of invoices or receipts therefor for reasonable meals, accommodations, and travel expenses, including economy class airfare (or other class consistent with the uniQure travel policy for leadership team members), and first-class train ticket. The Consultant shall submit to the Company an itemized statement, in a form satisfactory to the Company, of such expenses incurred in relation to the performance of the Services within 30 days following the last day of the month in which such expenses were incurred. The Company shall pay the Consultant the amount shown on such statement within 30 days after receipt. Notwithstanding the foregoing, expenses shall at all times be submitted and paid in accordance with the Company's travel and expense policies, which may be amended from time to time in the Company's sole discretion.

5. Benefits and Taxes. Except as provided for herein, the Consultant shall not be entitled in connection to the Services to any benefits, coverages, or privileges, including, without limitation, social security, unemployment, medical or pension payments, made available to employees of the Company. The Consultant shall be solely responsible for payment of all taxes,

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contributions or any other mandatory charges which, as a result of Services, may be or are imposed on Consultant. The Consultant warrants that he will make orderly payments of tax liabilities for the remuneration received from the Company under this Agreement. The Consultant hereby undertakes and guarantees to indemnify and hold the Company harmless from any and all related claims that may arise under this Section 5.

6. Cooperation. The Consultant shall use his best efforts in the performance of his obligations under this Agreement. The Company shall provide such access to its information and property as may be reasonably required to permit the Consultant to perform his obligations hereunder. The Consultant agrees to declare in a readily understandable way that he is a consultant to the Company whenever he writes or speaks in public about the Company or on any issue relating to the Company or its undertakings.

7. Proprietary Information, Privileged Information, and Personal Data.

(a) Proprietary Information. The Consultant acknowledges that his relationship with the Company is one of high trust and confidence and that during the term of this Agreement, he will have access to and contact with Proprietary Information. The Consultant agrees that he will not, during the Term or at any time, thereafter, disclose to others, or use for his benefit or the benefit of others, any Proprietary Information. For purposes of this Agreement, Proprietary Information shall mean, confidential, non-public and or proprietary business, clinical, technical and other information owned by or in the possession, custody or control of Company, including without limitation, formula, vendor information, customer information, apparatus, equipment, trade secret, process, research, report, clinical data, technical data, assays, know-how, computer program, software, software documentation, hardware design, technology, marketing or business plan, forecast, unpublished financial statement, budget, license, price, cost and employee list that is communicated to, learned of, developed or otherwise acquired by the Consultant in the course of his service as a consultant to the Company. The Company shall own any intellectual property generated using the Proprietary Information of the Company and any results and proceeds therefrom.

The Consultant's obligations under this Section 7(a) shall not apply to any information that (i) is or becomes known to the general public under circumstances involving no breach by the Consultant or others of the terms of this Section 7(a) or the Employment Agreement or surviving terms thereof, (ii) is generally disclosed to third parties by the Company without restriction on such third parties, (iii) is approved for release by written authorization of the Board of Directors of the Company, (iv) was known to the Consultant before the consulting services were performed except in connection with his prior service to the Company, (v) is rightfully disclosed to the Consultant without restriction by a third party, (vi) is independently developed by the Consultant without use of the Proprietary Information, or (vii) the Consultant is required to disclose by law, government regulation, or court order.

The Consultant represents that his retention as a consultant with the Company and his performance under this Agreement does not, and shall not, breach any agreement that obligates him to keep in confidence any trade secrets or confidential or proprietary information of his or of any other party, or to refrain from competing, directly or indirectly, with the business of any other

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party. The Consultant shall not disclose to the Company any trade secrets or confidential or proprietary information of any other party.

This Section 7(a) shall survive termination or expiration of this Agreement for a period of seven (7) years, except that any the non-disclosure obligation with respect to any information that could constitute a trade secret shall survive into perpetuity.

Nothing in this Agreement restricts or prohibits the Consultant from initiating communications directly with, responding to any inquiries from, providing testimony before, providing confidential information to, reporting possible violations of law or regulation to, or from filing a claim or assisting with an investigation directly with a self-regulatory authority or a government agency, or from making other disclosures that are protected under the whistleblower provisions of federal, state, or local law or regulation. The Consultant does not need the prior authorization of the Company to engage in conduct protected by this Section, and the Consultant does not need to notify the Company that he has engaged in such conduct. Notwithstanding any non-disclosure obligations that the Consultant may have pursuant to this Agreement or any other agreement with the Company, pursuant to 18 U.S.C. Section 1833(b), the Consultant shall not be held criminally or civilly liable under any federal or state trade secret law for the disclosure of a trade secret that is made: (i) in confidence to a federal, state, or local government official, either directly or indirectly, or to an attorney, and solely for the purpose of reporting or investigating a suspected violation of law; or (ii) in a complaint or other document filed in a lawsuit or other proceeding, if such filing is made under seal.

(b) Personal Data

(i) The Company processes the following personal data of its contractual partners, including the Consultant (as applicable): name, field of expertise, BIG registration number, place of work, compensation paid, hourly rate, and past experience of the Company with such contractual partner. This personal data will be processed for administrative, statistical, information and marketing purposes in accordance with the applicable legal provisions on data protection. Personal data of its contractual partners is used exclusively for these purposes by the Company, its members of the uniQure group and their employees. The use includes the transfer of data to other entities of the Company thereby including those companies which are located outside of the EEA where the legal protection of personal data may not be the same as in the Netherlands. The Consultant consents to the onward transfer and use of the Consultant's personal data to other entities of the uniQure group of companies located in the United States of America. The Consultant is free to withdraw his consent at any time. With respect to his personal data, the Consultant always has the right to access his personal data, and, in the event the data is incorrect or irrelevant considering the purposes of processing, the right to request correction, removal or blocking thereof as well as the right to raise objections against a processing of such data. Requests may be submitted to uniQure biopharma B.V., attn. Legal Counsel, Paasheuvelweg 25A, 1105 BP, Amsterdam.

(ii) In case the Services allow the Consultant access to data as defined in the applicable data protection legislation ("personal data"), the Consultant shall at all times:

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Lexington, MA 02421

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Paasheuvelweg 25A
P.O. Box 22506

- collect and process personal data in accordance with the provisions of this Agreement or as otherwise instructed by Company from time to time;

- ensure that any personal data relating to individuals other than the categories of data related to the specifications of the Services will not be collected;

- collect and process personal data solely for the purposes of the Services and in the manner specified in the specifications of the Services and not further to process such data in any other manner;

- collect and process personal data fairly and lawfully;

- not disclose personal data to any third party without the prior permission in writing of the Company, or where such disclosure is required by any local law, regulation or supervisory authority in which case the Consultant will, wherever possible, notify the Company prior to complying with any such request for disclosure and shall comply with all reasonable directions of the Company with respect to such disclosure;

- ensure that all personal data are accurate and, where necessary, kept up to date and use best efforts to ensure that trial data which is inaccurate or incomplete is erased or rectified;

- comply with all written instructions issued by the Company to de-identify the personal data from time to time;

- ensure that the Company is notified promptly (and in any event within five days of receipt) of any communication received from a subject relating to subject access rights;

- ensure that technical and organizational measures are taken to protect personal data against accidental or unlawful destruction or accidental loss or damage, alteration, or unauthorized disclosure, and against all other unauthorized disclosure or access and against all other unauthorized or unlawful forms of processing; and

- assist Company in complying with any applicable security breach notification duties; the Consultant shall report any breach of security leading to the accidental or unlawful destruction, loss, alteration, unauthorised disclosure of, or access to, personal data transmitted, stored, or otherwise processed to the applicable authority without the Company's prior written approval.

The covenants set forth in this Section 7 are in addition to, not in lieu of, any other covenants the Consultant has to the Company and/or its affiliates. To the maximum extent permitted under applicable law, the covenants shall be construed together to be as protective as possible of the legitimate business interests of the Company and its affiliates.

8. Representations.

(a) The Consultant will perform the Services in accordance with generally accepted professional standards as well as standards designated by the Company in its Code of Conduct and otherwise. The Consultant shall perform all work performed as part of the contractual relationship with the Company in a manner consistent with all applicable laws, regulations and

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standards including all applicable anti-bribery and antitrust laws. The Consultant has not made or provided, and will not make or provide, any payment or benefit, directly or indirectly, to government officials, customers, business partners, healthcare professionals or any other person to secure an improper benefit or unfair business advantage, affect private or official decision-making, affect prescription behavior, or induce someone to breach professional duties or standards.

(b) The Consultant will immediately report to the Company in writing any suspected or detected violation of the above principles in connection with the Company's business and, in such cases, will cooperate fully with the Company in reviewing the matter. If the Company believes in good faith that the Consultant has violated any of the above principles, the Company shall have the unilateral right to terminate the contractual relationship with immediate effect, as described in Section 3.

(c) The Consultant hereby represents that, except as the Consultant has disclosed in writing to the Company, the Consultant is not bound by the terms of any agreement with any third party to refrain from using or disclosing any trade secret or confidential or proprietary information in the course of the Consultant's consultancy with the Company, to refrain from competing, directly or indirectly, with the business of such third party or to refrain from soliciting employees, customers or suppliers of such third party. The Consultant further represents that his performance of all the terms of this Agreement and the performance of the Services as a consultant of the Company do not and will not breach any agreement with any third party to which the Consultant is a party (including, without limitation, any nondisclosure or non-competition agreement), or obligations to an employer, and that the Consultant will not disclose to the Company or induce the Company to use any confidential or proprietary information or material belonging to any current or previous employer or any other party. In the event of a conflict or potential conflict, the Consultant must disclose any actual or potential conflict to Company, and the Parties will reasonably cooperate to resolve any such conflict. If either party reasonably believes that the conflict cannot be resolved, that party may terminate this Agreement, and no further Services shall be performed.

(d) The Consultant represents and agrees that he holds and shall maintain all necessary licenses and insurance to furnish the Services contemplated by this Agreement.

(e) The Consultant hereby acknowledges that he, and any of his representatives, if applicable, will have access to material non-public information concerning the Company. Consultant acknowledges that he, and any of his representatives, if applicable, are aware, that the United States or other applicable securities laws prohibit any person, who has received from an issuer material non-public information relating to an issuer of securities, from purchasing or selling securities of such issuer or from communicating such information to any other person under circumstances in which it is reasonably foreseeable that such person is likely to purchase or sell such securities.

9. Intellectual Property Rights. The Consultant's rights, title and interest in inventions, discoveries and developments conceived or reduced to practice in the performance of Company-funded consulting services made by the Consultant (including any of its employees, agents, or representatives) whether solely or jointly with Company employees, agents or representatives

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("Inventions") shall be immediately assigned to Company, and, to the extent not immediately assigned, shall be disclosed by the Consultant and assigned in writing promptly upon the request of the Company. Additionally, all Work Product shall constitute a Work Made For Hire as so defined in the U.S. Copyright Act of 1976 (and any amendments thereto), which shall be made for the express benefit of the Company.

10. Restrictive Covenants. The Consultant reaffirms and agrees that he remains bound by the terms of the Confidentiality, Inventions, and Restrictive Covenants Agreement between him and the Company, dated on or about September 14, 2020 (the "CIRCA").

11. Notices. All notices required or permitted under this Agreement shall be in writing and shall be deemed effective upon personal delivery or upon deposit in the United States Post Office, by registered or certified mail, postage prepaid, addressed to the other Party at the address shown above, or at such other address or addresses, including electronic mail addresses, as either Party shall designate to the other in accordance with this Section 11. Additionally, a copy of all notices shall be sent to the Company at legalnotices@uniqure.com.

12. Use of Name. Neither Party shall use the name of the other Party, nor any variation thereon, nor adaptation thereof may be used in any advertising, promotional sales literature, or other publicity without the prior written approval of the Party whose name is to be used.

13. Pronouns. Whenever the context may require, any pronouns used in this Agreement shall include the corresponding masculine, feminine or neuter forms, and the singular forms of nouns and pronouns shall include the plural, and vice versa.

14. Entire Agreement. This Agreement constitutes the entire agreement between the Parties and supersedes all prior agreements and understandings, whether written or oral, relating to the subject matter of this Agreement, provided, however, that this Agreement does not alter or amend: (a) the surviving obligations under the Employment Agreement, (b) the CIRCA, or (c) the terms of any equity award granted to the Consultant from the Company or any affiliate of the Company on or before the Effective Date, except as set forth in Section 4(b).

15. Amendment. This Agreement may be amended or modified only by a written instrument executed by both the Company and the Consultant.

16. Governing Law/Forum Selection. This Agreement shall be construed, interpreted, and enforced in accordance with the laws of the Commonwealth of Massachusetts, without giving effect to the conflict or choice of law provisions thereof. Additionally, the state and federal courts sitting in the Commonwealth of Massachusetts shall be the sole and exclusive jurisdiction for any disputes arising out of this Agreement.

17. Successors and Assigns. This Agreement shall be binding upon, and inure to the benefit of, both Parties and their respective successors and assigns, including any corporation with which, or into which, the Company may be merged, or which may succeed to its assets or business, provided, however, that the obligations of the Consultant are personal and shall not be assigned by him.

18. Delegation and Assignment. The Consultant acknowledges that he is prohibited from subcontracting, delegating, or assigning any of his rights or obligations under this Agreement without the Company's prior written consent. The Company may assign its rights or obligations under this Agreement to a third party provided it will cause such party to be bound to the terms of this Agreement.

19. Remedies. The Consultant acknowledges that any breach of the provisions of Section 7 of this Agreement may result in serious and irreparable injury to the Company for which the Company may not be adequately compensated by monetary damages alone. The Consultant agrees, therefore, that, in addition to any other remedy it may have, the Company may be entitled to enforce the specific performance of this Agreement by the Consultant and to seek both temporary and permanent injunctive relief without the posting of a bond (to the extent permitted by law).

20. Miscellaneous.

(a) No delay or omission by the Company in exercising any right under this Agreement shall operate as a waiver of that or any other right. A waiver or consent given by the Company on any one occasion shall be effective only in that instance and shall not be construed as a bar or waiver of any right on any other occasion.

(b) The captions of the sections of this Agreement are for convenience of reference only and in no way define, limit, or affect the scope or substance of any section of this Agreement.

(c) In the event that any provision of this Agreement shall be invalid, illegal, or otherwise unenforceable, the validity, legality and enforceability of the remaining provisions shall in no way be affected or impaired thereby. The Parties shall negotiate a provision that comes closest to the desired purpose of the illegal, invalid, or unenforceable provision.

(d) For purposes of clarity, Sections 4, 5, 6, 7, 8, 9, 10, 11, 12, 16, and 19 shall survive termination of this Agreement.

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IN WITNESS WHEREOF, the Parties hereto have executed this Agreement as of the last day and year set forth below.

uniQure, Inc.

By: /s/ Matthew Kapusta

Matt Kapusta

Chief Executive Officer

Date: 10/4/2023

Consultant

By: /s/ Ricardo E. Dolmetsch Ph.D.

Ricardo E. Dolmetsch Ph.D.

Date: 10/4/2023

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Exhibit 31.1

Certification of Chief Executive Officer

I, Matthew Kapusta, certify that:

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

2. Based on my knowledge, this report does not contain any untrue statement of a material fact or omit to state a material fact necessary to make the statements made, in light of the circumstances under which such statements were made, not misleading with respect to the period covered by this report;

3. Based on my knowledge, the financial statements, and other financial information included in this report, fairly present in all material respects the financial condition, results of operations and cash flows of the registrant as of, and for, the periods presented in this report;

4. The registrant's other certifying officer and I are responsible for establishing and maintaining disclosure controls and procedures (as defined in Exchange Act Rules 13a-15(e) and 15d-15(e)) and internal control over financial reporting (as defined in Exchange Act Rules 13a-15(f) and 15d-15(f)) for the registrant and have:

(a) Designed such disclosure controls and procedures, or caused such disclosure controls and procedures to be designed under our supervision, to ensure that material information relating to the registrant, including its consolidated subsidiaries, is made known to us by others within those entities, particularly during the period in which this report is being prepared;

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

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

evaluation;

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

5. The registrant's other certifying officer and I have disclosed, based on our most recent evaluation of internal control over financial reporting, to the registrant's auditors and the audit committee of the registrant's board of directors (or persons performing the equivalent functions):

(a) All significant deficiencies and material weaknesses in the design or operation of internal control over financial reporting which are reasonably likely to adversely affect the registrant's ability to record, process, summarize and report financial information; and

(b) Any fraud, whether or not material, that involves management or other employees who have a significant role in the registrant's internal control over financial reporting.

By: /s/ MATTHEW KAPUSTA

Matthew Kapusta
Chief Executive Officer
(Principal Executive Officer)
November May 7, 2023 2024

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)
November May 7, 2023 2024

Exhibit 32.1

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

In connection with this Quarterly Report of uniQure N.V. (the "Company") on Form 10-Q for the period ended September 30, 2023 March 31, 2024, 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)

November May 7, 2023 2024

By: /s/ CHRISTIAN KLEMT

Christian Klemt

Chief Financial Officer
(Principal Financial Officer)

November May 7, 2023 2024

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.

DISCLAIMER

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