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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, 2026

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: 001-38520

MeiraGTx Holdings plc

(Exact Name of Registrant as Specified in its Charter)

Cayman Islands **(State or other jurisdiction of incorporation or organization)**	**98-1448305** **(I.R.S. Employer Identification No.)**
655 Third Avenue, Suite 1115 **New York, NY** **(Address of principal executive offices)**	**10017** **(Zip Code)**

Registrant's telephone number, including area code: (646) 860-7985

Not Applicable

(Former name, former address, and former fiscal year, if changed since last report)

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, $0.00003881 par value per share	MGTX	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," "smaller reporting company," and "emerging growth company" in Rule 12b-2 of the Exchange Act.

Large accelerated filer ☐	Accelerated filer ☐
Non-accelerated filer ☒	Small 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 4, 2026, the registrant had 97,730,540 ordinary shares, $0.00003881 par value per share, outstanding.

Forward-Looking Statements

This Quarterly Report on Form 10-Q (the "Form 10-Q") contains forward-looking statements within the meaning of the Private Securities Litigation Reform Act of 1995. All statements contained in this Form 10-Q that do not relate to matters of historical fact should be considered forward-looking statements, including, without limitation, statements regarding expectations regarding meetings with global regulatory authorities and the FDA, product pipeline, anticipated product benefits, goals and strategic transactions or priorities, product candidate development and status and expectations relating to clinical trials, growth expectations or targets, pre-clinical and clinical data expectations in respect of collaborations and expectations related to financing arrangements and the intended use of proceeds thereunder, as well as statements that include the words "expect," "will," "intend," "plan," "believe," "project," "forecast," "estimate," "may," "could," "should," "would," "continue," "anticipate," "eligible" and similar statements of a future or forward-looking nature. These forward-looking statements are based on management's current expectations. These statements are neither promises nor guarantees, but involve known and unknown risks, uncertainties and other important factors that may cause actual results, performance or achievements to be materially different from any future results, performance or achievements expressed or implied by the forward-looking statements, including, but not limited to, the important factors discussed under "Item 1A. Risk Factors" in this Form 10-Q. These and other important factors could cause actual results to differ materially from those indicated by the forward-looking statements made in this Form 10-Q. Any such forward-looking statements represent management's estimates as of the date of this Form 10-Q. While we may elect to update such forward-looking statements at some point in the future, unless required by law, we disclaim any obligation to do so, even if subsequent events cause our views to change. Thus, one should not assume that our silence over time means that actual events are bearing out as expressed or implied in such forward-looking statements. These forward-looking statements should not be relied upon as representing our views as of any date subsequent to the date of this Form 10-Q.

Risk Factor Summary

We are providing the following summary of the principal risk factors contained in this Form 10-Q to enhance the readability and accessibility of our risk factor disclosures. We encourage you to carefully review in their entirety the full risk factors set forth in the section of this Form 10-Q captioned "Part II—Item 1A. Risk Factors" for additional information regarding the material factors that make an investment in our ordinary shares speculative or risky. These risks and uncertainties include, among others, the following:

- We have incurred significant losses since inception and anticipate that we will incur continued losses for the foreseeable future, and may never achieve or maintain profitability.
- We will require additional capital to fund our operations, which may not be available on acceptable terms, if at all.
- We may not have sufficient cash flows or cash on hand to satisfy our debt obligations or covenants under our financing arrangements, or we may not be able to effectively manage our business in compliance with such covenants.
- Our review of potential strategic transactions may not result in an executed or consummated transaction or other strategic alternative and may not result in anticipated benefits to us or our shareholders, and the process of reviewing strategic transactions or its conclusion could be disruptive and distracting to our business operations and management.
- We are heavily dependent on the success of our product candidates, which are still in development, and if none of them receive regulatory approval or are successfully commercialized, our business may be harmed.
- It is difficult to predict the time and cost of product candidate development on our novel gene therapy platform. A limited number of gene therapies have been approved in the United States or in Europe.
- Because gene therapy is novel and the regulatory landscape that governs any product candidates we may develop is uncertain and may change, we cannot predict the time and cost of obtaining regulatory approval, if we receive it at all, for any product candidates we may develop.
- Clinical trials are expensive, time-consuming, difficult to design and implement, and involve an uncertain outcome. Further, we may encounter substantial delays in our clinical trials.
- The affected populations for our product candidates may be smaller than we or third parties currently project, which may affect the addressable markets for our product candidates.
- We and our contract manufacturers for plasmid are subject to significant regulation with respect to manufacturing our products. Our manufacturing facilities and the third-party manufacturing facilities which we rely on may not continue to meet regulatory requirements and have limited capacity.
- Enacted and future healthcare legislation may increase the difficulty and cost for us to obtain marketing approval of and commercialize our product candidates and may affect the prices we may set.
- We are subject to regulation and other legal obligations relating to data privacy and protection. Compliance with these requirements is complex and costly. The actual or perceived failure to comply with such obligations could materially harm our business.
- We face significant competition in an environment of rapid technological change, and there is a possibility that our competitors may achieve regulatory approval before us or develop therapies that are

safer or more advanced or effective than ours, which may harm our financial condition and our ability to successfully market or commercialize any product candidates we may develop.

- We depend on proprietary technology licensed from others. If we lose our existing licenses or are unable to acquire or license additional proprietary rights from third parties, we may not be able to continue developing our product candidates.

- If we are unable to obtain and maintain patent protection for our technology and product candidates or if the scope of the patent protection obtained is not sufficiently broad, we may not be able to compete effectively in our markets.

- We may need to increase or decrease the size of our organization, and we may experience difficulties in managing these organizational changes, which could disrupt our operations.

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

Preliminary Notes

Unless the context otherwise requires, references in this Form 10-Q to "MeiraGTx," "we," "us", "our" or "the Company" refer to MeiraGTx Holdings plc and its subsidiaries.

We have proprietary rights to trademarks, trade names and service marks appearing in this Form 10-Q that are important to our business. Solely for convenience, the trademarks, trade names and service marks may appear in this Form 10-Q without the ® and TM symbols, but any such references are not intended to indicate, in any way, that we forgo or will not assert, to the fullest extent under applicable law, our rights or the rights of the applicable licensors to these trademarks, trade names and service marks. All trademarks, trade names and service marks appearing in this Form 10-Q are the property of their respective owners.

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PART I—FINANCIAL INFORMATION

Item 1. Financial Statements.

MEIRAGTX HOLDINGS PLC AND SUBSIDIARIES
CONDENSED CONSOLIDATED BALANCE SHEETS
(unaudited)
(in thousands, except share and per share amounts)

	September 30, 2026	December 31, 2025
ASSETS		
CURRENT ASSETS:		
Cash and cash equivalents	$ 184,650	$ 65,931
Accounts receivable	1,748	—
Accounts receivable - related party	3,818	3,000
Unbilled receivables	597	—
Unbilled receivables - related party	17,214	—
Prepaid expenses	3,828	6,017
Tax incentive receivable	6,183	15,286
Other current assets	24,308	1,527
Total Current Assets	242,346	91,761
Property, plant and equipment, net	96,871	105,465
Intangible assets, net	17	578
Restricted cash	2,102	2,262
Other assets	1,371	1,147
Equity method and other investments	35,999	6,749
Right-of-use assets - operating leases, net	10,913	12,852
Right-of-use assets - finance leases, net	22,076	23,616
TOTAL ASSETS	$ 411,695	$ 244,430
LIABILITIES AND SHAREHOLDERS' EQUITY		
CURRENT LIABILITIES:		
Accounts payable	$ 10,907	$ 10,066
Accrued expenses	17,483	32,893
Lease obligations - operating leases, current	2,431	2,851
Lease obligations - finance leases, current	41	38
Deferred revenue, current	518	—
Deferred revenue - related party, current	4,740	1,776
Note payable, net, current	—	24,648
Corporate tax liability	2,198	—
Other current liabilities	14,084	50,283
Total Current Liabilities	52,402	122,555
Deferred revenue	427	—
Deferred revenue - related party	11,137	65,120
Lease obligations - operating leases	9,405	11,351
Lease obligations - finance leases	74	109
Asset retirement obligations	1,129	1,399
Note payable, net	121,900	49,689
TOTAL LIABILITIES	196,474	250,223
COMMITMENTS AND CONTINGENCIES (Note 13)		
SHAREHOLDERS' EQUITY (DEFICIT):		
Ordinary Shares, $0.00003881 par value, 1,288,327,750 authorized, 97,619,453 and 81,120,931 shares issued and outstanding as of September 30, 2026 and December 31, 2025, respectively	3	3
Capital in excess of par value	983,067	808,021
Treasury shares	(18,193)	—
Accumulated other comprehensive gain	3,232	2,406
Accumulated deficit	(753,028)	(816,223)
Non-controlling interest	140	—
Total Shareholders' Equity (Deficit)	215,221	(5,793)
TOTAL LIABILITIES AND SHAREHOLDERS' EQUITY (DEFICIT)	$ 411,695	$ 244,430

See Notes to Condensed Consolidated Financial Statements

MEIRAGTX HOLDINGS PLC AND SUBSIDIARIES
CONDENSED CONSOLIDATED STATEMENTS OF OPERATIONS AND COMPREHENSIVE (LOSS) INCOME
(unaudited)
(in thousands, except share and per share amounts)

	For the Three-Month Periods Ended September 30,		For the Nine-Month Periods Ended September 30,	
	2026	2025	2026	2025
Revenues:				
Service revenue	$ 1,571	$ —	$ 13,456	$ —
Service revenue - related party	3,243	410	108,442	6,027
License revenue - related party	—	—	204,643	—
Total revenue	4,814	410	326,541	6,027
Operating expenses:				
Cost of service revenue	617	—	2,018	—
Cost of service revenue - related party	2,806	313	8,767	4,367
General and administrative	11,953	13,616	32,894	35,293
Research and development	39,316	32,532	129,132	98,807
Total operating expenses	54,692	46,461	172,811	138,467
(Loss) income from operations	(49,878)	(46,051)	153,730	(132,440)
Other non-operating (expense) income:				
Foreign currency (loss) gain	(1)	(1,558)	(4,304)	10,753
Interest income	1,150	161	2,045	1,540
Financial related costs, net	1,635	(3,065)	(4,722)	(9,142)
Other income (expense)	355	—	(4,868)	—
Loss on equity method investee	(3,929)	—	(75,831)	—
Income tax expense	(613)	—	(2,975)	—
Net (loss) income	$ (51,281)	$ (50,513)	$ 63,075	$ (129,289)
Net (loss) income attributed to:				
Net (loss) income attributed to shareholders	(51,212)	(50,513)	63,195	(129,289)
Net loss attributed to non-controlling interest	(69)	—	(120)	—
Net (loss) income	$ (51,281)	$ (50,513)	$ 63,075	$ (129,289)
Other comprehensive (loss) gain:				
Foreign currency translation (loss) gain attributed to shareholders	(23)	1,213	826	(2,593)
Foreign currency translation loss attributed to non-controlling interest	—	—	(4)	—
Comprehensive (loss) income	$ (51,304)	$ (49,300)	$ 63,897	$ (131,882)
Net (loss) income per ordinary share:				
Basic	$ (0.53)	$ (0.62)	$ 0.70	$ (1.61)
Diluted	$ (0.53)	$ (0.62)	$ 0.68	$ (1.61)
Weighted-average number of ordinary shares outstanding:				
Basic	96,879,809	80,947,703	89,923,492	80,195,572
Diluted	96,879,809	80,947,703	93,446,129	80,195,572

See Notes to Condensed Consolidated Financial Statements

MEIRAGTX HOLDINGS PLC AND SUBSIDIARIES
CONDENSED CONSOLIDATED STATEMENT OF SHAREHOLDERS' EQUITY
FOR THE PERIOD ENDED SEPTEMBER 30, 2026
(unaudited)
(in thousands, except share amounts)

	Ordinary Shares	Amount	Capital in Excess of Par Value	Treasury Shares	Accumulated Other Comprehensive Gain	Accumulated Deficit	Non-Controlling Interest	Total Shareholders' Deficit
Balance at December 31, 2025	81,120,931	$ 3	$ 808,021	$ —	$ 2,406	$ (816,223)	$ —	$ (5,793)
Share-based compensation activity	824,466	—	(2,244)	—	—	—	—	(2,244)
Issuance of ordinary shares in at-the-market offering	1,770,729	—	14,027	—	—	—	—	14,027
Issuance costs in connection with ordinary shares	—	—	(35)	—	—	—	—	(35)
Repurchase of treasury shares	(2,300,000)	—	—	(18,193)	—	—	—	(18,193)
Issuance of ordinary shares in connection with license agreement	30,000	—	249	—	—	—	—	249
Other comprehensive gain	—	—	—	—	172	—	—	172
Net loss for the three-month period ended March 31, 2026	—	—	—	—	—	(46,313)	—	(46,313)
Balance at March 31, 2026	81,446,126	3	820,018	(18,193)	2,578	(862,536)	—	(58,130)
Share-based compensation activity	237,245	—	4,545	—	—	—	—	4,545
Issuance of ordinary shares in at-the-market offering	1,393,636	—	14,180	—	—	—	—	14,180
Issuance of ordinary shares in connection with registered offering	11,111,111	—	100,000	—	—	—	—	100,000
Contributions from non-controlling interest	—	—	—	—	—	—	3	3
Sale of non-controlling interest	—	—	412	—	—	—	261	673
Issuance costs in connection with ordinary shares	—	—	(6,832)	—	—	—	—	(6,832)
Other comprehensive gain (loss)	—	—	—	—	677	—	(4)	673
Net income (loss) for the three-month period ended June 30, 2026	—	—	—	—	—	160,720	(51)	160,669
Balance at June 30, 2026	94,188,118	3	932,323	(18,193)	3,255	(701,816)	209	215,781
Share-based compensation activity	70,162	—	8,280	—	—	—	—	8,280
Issuance of ordinary shares in at-the-market offering	2,410,603	—	32,551	—	—	—	—	32,551
Issuance of shares in connection with Royalty Note Purchase Agreement	950,570	—	10,000	—	—	—	—	10,000
Contributions from non-controlling interest	—	—	—	—	—	—	—	—
Issuance costs in connection with ordinary shares	—	—	(87)	—	—	—	—	(87)
Other comprehensive loss	—	—	—	—	(23)	—	—	(23)
Net loss for the three-month period ended September 30, 2026	—	—	—	—	—	(51,212)	(69)	(51,281)
Balance at September 30, 2026	97,619,453	$ 3	$ 983,067	$ (18,193)	$ 3,232	$ (753,028)	$ 140	$ 215,221

See Notes to Condensed Consolidated Financial Statements

MEIRAGTX HOLDINGS PLC AND SUBSIDIARIES
CONDENSED CONSOLIDATED STATEMENT OF SHAREHOLDERS' EQUITY (DEFICIT)
FOR THE PERIOD ENDED SEPTEMBER 30, 2025
(unaudited)
(in thousands, except share amounts)

	Ordinary Shares	Amount	Capital in Excess of Par Value	Accumulated Other Comprehensive Loss	Accumulated Deficit	Total Shareholders' Equity
Balance at December 31, 2024	78,397,380	$ 3	$ 773,565	$ (3,719)	$ (702,022)	$ 67,827
Share-based compensation activity	457,679	—	2,130	—	—	2,130
Issuance of ordinary shares in at-the-market offering	563,379	—	4,482	—	—	4,482
Issuance costs in connection with ordinary shares	—	—	(12)	—	—	(12)
Other comprehensive loss	—	—	—	(1,347)	—	(1,347)
Net loss for the three-month period ended March 31, 2025	—	—	—	—	(39,981)	(39,981)
Balance at March 31, 2025	79,418,438	3	780,165	(5,066)	(742,003)	33,099
Share-based compensation activity	80,925	—	5,696	—	—	5,696
Issuance of ordinary shares in at-the-market offering	946,921	—	5,433	—	—	5,433
Issuance costs in connection with ordinary shares	—	—	(14)	—	—	(14)
Other comprehensive loss	—	—	—	(2,459)	—	(2,459)
Net loss for the three-month period ended June 30, 2025	—	—	—	—	(38,795)	(38,795)
Balance at June 30, 2025	80,446,284	3	791,280	(7,525)	(780,798)	2,960
Share-based compensation activity	33,400	—	5,733	—	—	5,733
Issuance costs in connection with ordinary shares	—	—	50	—	—	50
Other comprehensive gain	—	—	—	1,213	—	1,213
Net loss for the three-month period ended September 30, 2025	—	—	—	—	(50,513)	(50,513)
Balance at September 30, 2025	80,479,684	$ 3	$ 797,063	$ (6,312)	$ (831,311)	$ (40,557)

See Notes to Condensed Consolidated Financial Statements

MEIRAGTX HOLDINGS PLC AND SUBSIDIARIES
CONDENSED CONSOLIDATED STATEMENTS OF CASH FLOWS
(unaudited)
(in thousands)

	For the Nine-Month Periods Ended September 30,	
	2026	2025
Cash flows from operating activities:		
Net income (loss)	$ 63,075	$ (129,289)
Adjustments to reconcile net income (loss) to net cash used in operating activities:		
Share-based compensation expense	15,096	16,504
Foreign currency loss (gain)	4,304	(10,753)
Depreciation and amortization	8,680	9,427
Change in right-of-use assets	1,891	(2,209)
Gain on termination of lease liabilities	—	(1,465)
Loss on disposal of equipment, furniture and fixtures	1,503	310
Undistributed losses of equity method investee	75,831	—
Amortization of interest on asset retirement obligations	113	126
Change in asset retirement obligation	(81)	—
Amortization of debt discount	663	834
Non-cash consideration received from related party - revenue arrangement	(104,988)	—
Non-cash other income	4,861	—
Non-cash financing related costs	(3,100)	—
Acquired in-process research and development	27,728	—
(Increase) decrease in operating assets:		
Accounts receivable	(1,730)	—
Accounts receivable - related party	(4,206)	(2,143)
Unbilled receivables	(599)	—
Unbilled receivables - related party	(17,031)	—
Contract assets - related party	—	986
Prepaid expenses	2,202	(113)
Tax incentive receivable	8,630	163
Other current assets	(23,175)	2,357
Other assets, net	29	(32)
Increase (decrease) in operating liabilities:		
Accounts payable	117	(2,362)
Accrued expenses	(15,421)	(4,329)
Lease liabilities	(2,281)	2,136
Corporate tax liability	2,198	—
Other current liabilities	(39,472)	27,557
Deferred revenue	946	—
Deferred revenue - related party	(51,059)	(695)
Net cash used in operating activities	(45,276)	(92,990)
Cash flows from investing activities:		
Purchase of property, plant and equipment	(4,072)	(3,554)
Acquisition of in-process research and development assets	(24,228)	—
Proceeds from sale of non-controlling interest	674	—
Proceeds from sale of equipment, furniture and fixtures	—	536
Net cash used in investing activities	(27,626)	(3,018)
Cash flows from financing activities:		
Exercise of share options	1,445	46
Payments of withholdings on shares withheld for income taxes	(5,960)	(2,991)
Payments on lease obligations - financing leases	(43)	—
Proceeds from the issuance of ordinary shares	170,758	9,915
Issuance costs in connection with ordinary shares	(6,455)	(26)
Payment for treasury shares	(18,193)	—
Contributions from non-controlling interest	3	—
Payment of notes payable	(75,000)	—
Proceeds from royalty notes payable	125,000	—
Net cash provided by financing activities	191,555	6,944
Net increase (decrease) in cash, cash equivalents and restricted cash	118,653	(89,064)
Effect of exchange rate changes on cash, cash equivalents and restricted cash	(94)	502
Cash, cash equivalents and restricted cash at beginning of the period	68,193	105,668
Cash, cash equivalents and restricted cash at end of the period	$ 186,752	$ 17,106
Supplemental disclosure of non-cash transactions:		
Non-cash contribution for acquisition of in-process research and development	$ 3,263	$ —
Issuance of shares in connection with a license agreement	$ 249	$ —
Fixed asset acquisitions included in accounts payable and accrued expenses	$ 172	$ 753
Right-of-use assets obtained in exchange for lease liabilities	$ —	$ 8,200
Change in estimate of asset retirement obligation	$ 290	$ 1,476
Issuance costs in connection with sale of ordinary shares in accounts payable and accrued expenses at end of period	$ 499	$ 50
Supplemental disclosure of cash flow information:		
Cash paid for interest	$ 7,878	$ 8,275
Cash paid for income taxes	$ 498	$ —

See Notes to Condensed Consolidated Financial Statements

MEIRAGTX HOLDINGS PLC AND SUBSIDIARIES
NOTES TO CONDENSED CONSOLIDATED FINANCIAL STATEMENTS

1. Organization and Basis of Presentation

The Company

MeiraGTx Holdings plc and subsidiaries (the "Company" or "MeiraGTx Holdings"), an exempted company incorporated under the laws of the Cayman Islands, is a vertically integrated, clinical-stage genetic medicines company with a broad pipeline of four late-stage clinical programs. Each of these programs uses local delivery of small doses, resulting in disease-modifying effects in both inherited and more common diseases, in the eye, radiation-induced xerostomia and Parkinson's disease. The Company uses its innovative technology in optimization of capsids, promoters, and novel translational control elements to develop best-in-class, potent, safe viral vectors. The Company's broad pipeline is supported by end-to-end in-house manufacturing. The Company has built the most comprehensive manufacturing capabilities in the industry, including two that are licensed for good manufacturing practices ("GMP") viral vector production and a GMP Quality Control facility with clinical and commercial licensure. In addition, the Company has developed a proprietary manufacturing platform process over 10 years based on more than 20 different viral vectors with leading yield and quality aspects and commercial readiness. Uniquely, the Company has developed a novel technology for *in vivo* delivery of any biologic therapeutic using oral small molecules. This transformative riboswitch gene regulation platform technology allows precise, dose-responsive expression of gene expression by oral small molecules. The Company is focusing the riboswitch platform on regulated *in vivo* delivery of metabolic peptides, including leptin, GLP-1, GIP, glucagon, amylin and PYY, as well as cell therapy, CAR-T for liquid and solid tumors and autoimmune diseases, and additionally, PNS targets addressing long-term intractable pain. The Company has developed the technology to apply genetic medicine to common diseases, increasing efficacy, addressing novel targets, and expanding access in some of the largest disease areas where the unmet need remains high.

Asset Purchase and Related Agreements with Janssen Pharmaceuticals, Inc.

On April 15, 2026 ("Janssen Closing Date"), the Company and MeiraGTx Ocular entered into an Asset Purchase Agreement (the "Asset Purchase Agreement") with Janssen Pharmaceuticals, Inc. ("Janssen") to reacquire the global rights to botaretigene sparoparvovec ("bota-vec") for the treatment of X-linked retinitis pigmentosa associated with mutations in the *RPGR* gene (the "RPGR Product") through the acquisition of the license agreement and related assets associated with the development, manufacture and commercialization of the RPGR Product. The Company paid Janssen an upfront cash purchase price of $25.0 million, regaining full ownership and control of the RPGR Product and all associated future development and commercialization rights.

In addition to the upfront payment, the Asset Purchase Agreement includes a one-time contingent milestone payment of $50.0 million, payable upon the achievement of both of the following regulatory and commercial milestones: U.S. regulatory approval of the RPGR Product and cumulative U.S. net sales exceeding $250.0 million. The Company is also obligated to pay Janssen tiered royalties in the mid-teens on future global net sales of RPGR Products beginning on or after July 1, 2029. If the Company enters into future licensing or commercialization arrangements for the RPGR Product with third parties, Janssen is also entitled to receive a portion of certain upfront and milestone payments, as well as royalties based on amounts received from such third parties or, in certain cases, on the third party's net sales of RPGR Products.

As part of the transaction, the parties entered into a Termination Agreement on the Janssen Closing Date (the "Termination Agreement") to terminate the prior asset purchase agreement (the "Original Asset Purchase Agreement") pursuant to which the Company and MeiraGTx UK II sold the RPGR Product to Janssen, and the related supply agreement (the "Supply Agreement"), each entered into on December 20, 2023, thereby restoring the Company's full strategic and operational control over the RPGR Product while establishing the future contingent payment obligations described above.

Johnson & Johnson Innovation – JJDC, Inc. (“JJDC”), the investment arm of Johnson & Johnson and the indirect owner of Janssen, continues to hold more than 5% of the Company's outstanding ordinary shares. As a result, Janssen remains a related party of the Company for financial reporting purposes. In connection with the above transaction, JJDC and Janssen have also agreed not to sell or transfer any of the Company’s ordinary shares or securities convertible into, exchangeable for, or exercisable for the Company’s ordinary shares, for twelve months following the Janssen Closing Date and following such twelve month period, if they ever intend to sell the Company’s shares after the twelve month period, they will provide written notice to the Company at least five business days prior to taking any action.

Eli Lilly and Company Collaboration Agreement

On November 7, 2025 (the “Lilly Effective Date”), MeiraGTx Ocular, MeiraGTx Limited and MeiraGTx UK II (collectively, “MeiraGTx”), entered into a strategic collaboration and license agreement with Eli Lilly and Company (“Lilly”) (the “Lilly Collaboration Agreement”) for the research, development and commercialization of genetic medicines in and related to the area of ophthalmology. Under the Lilly Collaboration Agreement, MeiraGTx has granted Lilly exclusive, worldwide rights to research, develop and commercialize the Company’s product candidate AAV-AIPL1, which treats Leber congenital amaurosis 4, or LCA4, caused by mutations in the AIPL1 gene, as well as two other preclinical product candidates which are intended to treat other inherited retinal dystrophies. As of the Lilly Effective Date, Lilly has (i) an exclusive license to proprietary intravitreal capsids for use with up to five targets, relating to or useful in the field of ophthalmology, to be selected by Lilly, (ii) an exclusive license to proprietary pan-retinal or rod-specific promoters for use with up to five targets, relating to or useful in the field of ophthalmology, to be selected by Lilly and (iii) a right of first designation with respect to certain target-specific transactions that MeiraGTx Ocular or its affiliates may seek to pursue in the field of ophthalmology. Lilly also has a right of first negotiation for use of the Company’s proprietary riboswitch technology in the field of ophthalmological gene editing.

Under the terms of the Lilly Collaboration Agreement, MeiraGTx received an upfront payment of $75.0 million after signing the Lilly Collaboration Agreement and will be eligible to receive up to over $400.0 million in total milestone payments, including up to $135.0 million in other potential near-term cash consideration upon the achievement of certain development and regulatory approval milestones. Lilly has the right to research, develop and commercialize products under the Lilly Collaboration Agreement, at its own cost. The Company may also perform research, development and manufacturing services for Lilly under the Lilly Collaboration Agreement and related agreements, for which it is entitled to fees in accordance with the applicable contractual terms. The Lilly Collaboration Agreement also provides for tiered royalties to be paid to MeiraGTx Ocular on future product sales.

Hologen Strategic Collaboration

On March 9, 2025, the Company and certain of its affiliates entered into a strategic collaboration with Hologen Limited (“Hologen”) and certain of its affiliates to advance the development of certain central nervous system gene therapy programs and to support the Company’s manufacturing capabilities. Hologen is a leading developer of multi-modal generative AI foundation models of real-world clinical data for clinical medicine and pharmaceutical drug development. Under the collaboration, Hologen committed to provide an upfront payment of $200.0 million and up to an additional $230.0 million to fund the development of the Company’s AAV-GAD program for the treatment of Parkinson’s disease to commercialization and other locally delivered central nervous system therapies. The Company also received an aggregate of 500,000 Class A shares of Hologen for nominal consideration. As of September 30, 2026, Hologen had funded $105.0 million of the upfront payment commitment.

The collaboration is governed by two framework agreements. The first, between the Company, MeiraGTx Neuro UK, Reogen Limited (formerly known as Hologen Neuro AI Limited) (“Reogen”) and Hologen, relates to the management of the business and affairs of Reogen, including the research, development, manufacture and commercialization of our (i) AAV-GAD investigational gene therapy for the treatment of Parkinson’s disease, AAV-BDNF investigational gene therapy for the treatment of genetic obesity disorders and other potential locally delivered genetic medicines to the central nervous system (the “Clinical Programs”) and (ii) proprietary device designed to effect the local delivery of a gene therapy product into the central nervous system or any topographic or

subcutaneous tissue modification on the face and scalp, of humans or animals (the "Delivery Device"), in each case, in accordance with the terms and conditions of the Reogen Collaboration Agreement as further described below. The second, between MeiraGTx Manufacturing, MeiraGTx Limited and Hologen, provides for the management of the business and affairs of MeiraGTx Manufacturing, including Hologen's investment in MeiraGTx Manufacturing and its participation in funding the manufacturing business.

During the second quarter of 2026, the parties completed the initial closing of the strategic collaboration and entered into amendments to the framework agreements to facilitate the initial funding and investment structures. As part of the initial closing, the Company, MeiraGTx Neuro UK, MeiraGTx Neuro US, Reogen, Hologen Neuro AI UK Limited and Hologen entered into the Collaboration and License Agreement, dated as of April 20, 2026 (the "Reogen Collaboration Agreement") governing the research, development, manufacturing and commercialization of the Clinical Programs and the Delivery Device. The parties also completed Hologen's initial investment in MeiraGTx Manufacturing. Following the initial closing, MeiraGTx Neuro UK holds a 39% ownership interest in Reogen, and Hologen holds a minority interest in MeiraGTx Manufacturing.

Following the initial closing, Hologen will fund an additional $95.0 million to satisfy the remaining portion of the upfront payments provided for under the framework agreements and the Reogen Collaboration Agreement. These funds will be used by Hologen to purchase (i) a portion of the Reogen shares held by MeiraGTx Neuro UK, such that following the purchase, MeiraGTx Neuro UK and Hologen are expected to hold 30% and 70% of the outstanding shares in Reogen, respectively, and (ii) additional shares in MeiraGTx Manufacturing from MeiraGTx Limited, such that following the purchase, Hologen will have increased its minority interest in MeiraGTx Manufacturing. The Company's obligation to issue additional MeiraGTx Manufacturing shares in exchange for the additional funding gives rise to a contingent forward financial instrument, which is recognized and measured at fair value as described in Note 5. The framework agreement relating to MeiraGTx Manufacturing also provides Hologen with an option to increase its ownership interest in MeiraGTx Manufacturing to up to 40%, which may be exercised within twelve months after Hologen purchases all of its additional shares in MeiraGTx Manufacturing using a portion of the proceeds from the remaining amount of the upfront payment to be paid, subject to the terms and conditions of the framework agreement. This option gives rise to a financial instrument that is recognized and measured at fair value as described in Note 5. If Hologen does not exercise the option during that twelve month period, then the Company has an option to purchase all of the shares of MeiraGTx Manufacturing held by Hologen for the same price that Hologen paid for such shares. The Company may exercise its option beginning on the third anniversary of the date Hologen purchases its additional shares in MeiraGTx Manufacturing and ending three years thereafter.

On September 29, 2026, the Company, MeiraGTx, LLC, MeiraGTx Neuro UK, MeiraGTx Neuro US, Reogen, Hologen Neuro AI UK Limited and Hologen entered into an Amendment to the Collaboration and License Agreement and Device Assignment and Assumption Agreement (the "Device Assignment Agreement"), pursuant to which the Company assigned certain intellectual property relating to, and contractual rights associated with the development of, the Delivery Device to Reogen, and amended the Reogen Collaboration Agreement to reflect the foregoing.

Basis of Presentation

The accompanying condensed consolidated financial statements have been prepared in conformity with accounting principles generally accepted in the United States of America ("GAAP"). Any reference in these notes to applicable guidance is meant to refer to the authoritative United States generally accepted accounting principles as found in the Accounting Standards Codification ("ASC") and Accounting Standards Updates ("ASU") of the Financial Accounting Standards Board ("FASB"). Certain reclassifications of prior period activities have been made to conform to current year presentation.

Interim Financial Statements

The accompanying condensed consolidated financial statements have been prepared in accordance with GAAP for interim financial information and with the instructions to Form 10-Q and Article 10 of Regulation S-X. Accordingly, they do not include all the information and footnotes required by GAAP for complete consolidated financial

statements. In the opinion of management, the condensed consolidated financial statements include all adjustments (consisting of normal recurring adjustments) necessary in order to make the condensed consolidated financial statements not misleading. Operating results for the nine-month period ended September 30, 2026 are not necessarily indicative of the results that may be expected for the year ending December 31, 2026. These unaudited condensed consolidated financial statements should be read in conjunction with the audited consolidated financial statements and the notes thereto for the year ended December 31, 2025 included in the Company's Annual Report on Form 10-K for the fiscal year ended December 31, 2025 (the "Form 10-K").

Liquidity

The Company does not currently have any approved products and has never generated any revenue from product sales. Although the Company has achieved profitable operations during the nine months ended September 30, 2026, such profitability was primarily attributable to non-recurring transactions and there is no assurance that profitable operations could be sustained on a continuing basis. In addition, development activities, clinical and preclinical testing, and commercialization of the Company's product candidates will require significant additional financing. The Company's accumulated deficit at September 30, 2026 totaled $753.0 million, and management expects to incur substantial losses in future periods. The success of the Company is subject to certain risks and uncertainties, including, among others: uncertainty of product development; competition in the Company's field of use; uncertainty of capital availability; uncertainty in the Company's ability to enter into agreements and consummate transactions with collaborative partners; expanding and protecting the Company's intellectual property portfolio; dependence on third parties; and dependence on key personnel. For the nine months ended September 30, 2026, the Company had $45.3 million cash flows used in operations. There are no assurances that the Company will generate positive cash flows in the future. Additionally, there are no assurances that the Company will be successful in obtaining an adequate level of financing for the development and commercialization of its product candidates.

As of September 30, 2026, the Company had cash, cash equivalents and restricted cash in the amount of $186.8 million, which consisted of depository and money market accounts held at large international banks. The Company estimates that its cash and cash equivalents on-hand, accounts receivable, accounts receivable – related party, unbilled receivables – related party, unbilled receivables and tax incentive receivable at September 30, 2026, will be sufficient to cover its expenses for at least the next twelve months from the date of issuance of these condensed consolidated financial statements. This estimate does not include the additional $95.0 million upfront payment from Hologen or the $135.0 million in potential near-term cash consideration from Lilly upon achievement of certain development and regulatory approval milestones, or any subsequent tranches available under the Royalty Note Purchase Agreement.

Risks and Uncertainties

The Company operates in an industry that is subject to intense competition, government regulation and rapid technological change. The Company's operations are subject to significant risk and uncertainties including financial, operational, technological, regulatory and other risks, including the potential risk of business failure.

The Company's capital resources and operations to date have been funded primarily with the proceeds from the Company's collaboration and business development activities and private and public equity offerings, as well as proceeds from debt financings. In the future, the Company may seek to raise additional capital through equity offerings, debt financings, marketing and distribution arrangements and other collaborations, strategic alliances and licensing arrangements or other sources to enable it to complete the development and potential commercialization of its product candidates.

2. Summary of Significant Accounting Policies and Recent Accounting Pronouncements

Certain of the Company's significant accounting policies are described below. All of the Company's significant accounting policies are disclosed in the notes to the audited consolidated financial statements as of and for the year ended December 31, 2025 included in the Company's Form 10-K.

Consolidation

The accompanying condensed consolidated financial statements include the accounts of MeiraGTx Holdings and its subsidiaries:

- MeiraGTx Limited, a limited company incorporated under the laws of England and Wales;
- MeiraGTx, LLC, a Delaware limited liability company;
- MeiraGTx UK II Limited, a limited company incorporated under the laws of England and Wales ("MeiraGTx UK II");
- MeiraGTx Ireland DAC, a designated activity company incorporated under the laws of Ireland ("MeiraGTx Ireland");
- MeiraGTx UK Limited, a limited company incorporated under the laws of England and Wales ("MeiraGTx UK");
- MeiraGTx Belgium, a private company with limited liability incorporated under the laws of Belgium ("MeiraGTx Belgium");
- MeiraGTx Cell Therapies, a simplified joint stock company, incorporated under the laws of France;
- BRI-Alzan, Inc., a Delaware corporation;
- MeiraGTx Bio, Inc., a Delaware corporation;
- MeiraGTx B.V., a private company with limited liability incorporated under the laws of the Netherlands ("MeiraGTx B.V.");
- MeiraGTx Netherlands, B.V., a private company with limited liability incorporated under the laws of the Netherlands ("MeiraGTx Netherlands");
- MeiraGTx Neurosciences, Inc., a Delaware corporation;
- MeiraGTx Therapeutics, Inc., a Delaware corporation;
- MeiraGTx Neuro I, LLC, a Delaware limited liability company ("MeiraGTx Neuro US");
- MeiraGTx Neuro II, LLC, a Delaware limited liability company;
- MeiraGTx Manufacturing Limited, a limited company incorporated under the laws of England and Wales ("MeiraGTx Manufacturing");
- MeiraGTx Ocular UK Limited, a limited company incorporated under the laws of England and Wales ("MeiraGTx Ocular");
- MeiraGTx Gene Regulation Limited, a limited company incorporated under the laws of England and Wales; and
- MeiraGTx Neuro UK Limited, a limited company incorporated under the laws of England and Wales ("MeiraGTx Neuro UK").

All the above subsidiaries are wholly owned by MeiraGTx Holdings, except that Hologen holds a minority interest in MeiraGTx Manufacturing, which wholly owns MeiraGTx UK II and MeiraGTx Ireland. All intercompany balances and transactions between the consolidated companies have been eliminated in consolidation.

Use of Estimates

Management considers many factors in selecting appropriate financial accounting policies and controls, and in developing the estimates and assumptions that are used in the preparation of these condensed consolidated financial statements. Management must apply significant judgment in this process. In addition, other factors may affect

estimates, including expected business and operational changes, sensitivity and volatility associated with the assumptions used in developing estimates, and whether historical trends are expected to be representative of future trends. The estimation process often may yield a range of potentially reasonable estimates of the ultimate future outcomes and management must select an amount that falls within that range of reasonable estimates. This process may result in actual results differing materially from those estimated amounts used in the preparation of the financial statements if these results differ from historical experience, or other assumptions do not turn out to be substantially accurate, even if such assumptions are reasonable when made. In preparing these condensed consolidated financial statements, management is required to make estimates and assumptions that affect the amounts reported in the condensed consolidated financial statements and accompanying notes. Significant estimates include, among others, the measurement of progress towards satisfaction of performance obligations, estimates of stand-alone selling price and material rights in revenue arrangements, the accounting for research and development costs, share-based compensation, lease related assumptions, asset retirement obligations, the fair value of financial instruments and the valuation of tax incentive receivable.

Restricted Cash

Restricted cash represents a guarantee put in place as required by the terms of the research and innovation grant from IDA Ireland which offers financial assistance in establishing the Company's operations in Shannon, Ireland. The following table provides a reconciliation of the components of cash and cash equivalents and restricted cash reported in the Company's condensed consolidated balance sheets to the total of the amount presented in the condensed consolidated statements of cash flows (in thousands):

	September 30, 2026	December 31, 2025
Cash and cash equivalents	$ 184,650	$ 65,931
Restricted cash	2,102	2,262
Total cash, cash equivalents and restricted cash in the condensed consolidated statements of cash flows	$ 186,752	$ 68,193

Fair Value Measurements

Fair value is defined as the price that would be received upon sale of an asset or paid upon transfer of a liability in an orderly transaction between market participants at the measurement date and in the principal or most advantageous market for that asset or liability. The fair value should be calculated based on assumptions that market participants would use in pricing the asset or liability, not on assumptions specific to the entity. In addition, the fair value of liabilities should include consideration of non-performance risk including the Company's own credit risk.

The Company follows ASC 820, *Fair Value Measurements and Disclosures*, for application to financial assets and liabilities. In addition to defining fair value, the standard expands the disclosure requirements around fair value and establishes a fair value hierarchy for valuation inputs. The hierarchy prioritizes the inputs into three levels based on the extent to which inputs used in measuring fair value are observable in the market. Each fair value measurement is reported in one of the three levels which are determined by the lowest level input that is significant to the fair value measurement in its entirety. These levels are:

- Level 1: Observable inputs such as quoted prices in active markets for identical assets the reporting entity has the ability to access as of the measurement date;
- Level 2: Inputs, other than the quoted prices in active markets, that are observable either directly or indirectly; and
- Level 3: Unobservable inputs in which there is little or no market data, which require the reporting entity to develop its own assumptions.

The table below represents the values of the Company's financial assets and liabilities that are required to be measured at fair value on a recurring basis (in thousands):

	Financial Asset (Liability)	Fair Value Measurement Using:		
Description	September 30, 2026	Significant Observable Inputs (Level 1)	Significant Other Observable Inputs (Level 2)	Significant Unobservable Inputs (Level 3)
Cash equivalents	$ 158,244	$ 158,244	$ —	$ —
Contingent forward instrument (Note 5)	$ 21,000	$ —	$ —	$ 21,000
Royalty Notes (Note 12)	$ (121,900)	$ —	$ —	$ (121,900)
Derivative liability (Note 5)	$ (4,433)	$ —	$ —	$ (4,433)
Equity option instrument (Note 5)	$ (2,085)	$ —	$ —	$ (2,085)

	Financial Asset (Liability)	Fair Value Measurement Using:		
Description	December 31, 2025	Significant Observable Inputs (Level 1)	Significant Other Observable Inputs (Level 2)	Significant Unobservable Inputs (Level 3)
Cash equivalents	$ 60,253	$ 60,253	$ —	$ —

	Contingent forward instrument	Derivative liability	Equity option instrument	Royalty Notes
Level 3 assets (liabilities), January 1, 2026	$ —	$ —	$ —	$ —
Issuance of instruments	$ 21,800	$ (5,223)	$ (2,450)	$ —
Purchase of Royalty Notes	$ —	$ —	$ —	$ (125,000)
Changes in fair value	$ (800)	$ 790	$ 365	$ 3,100
Level 3 assets (liabilities), September 30, 2026	$ 21,000	$ (4,433)	$ (2,085)	$ (121,900)

The Company's Level 3 financial instruments consist of the Royalty Notes, the contingent forward instrument, the equity option instrument and the derivative liability. These instruments are classified within Level 3 of the fair value hierarchy because their fair values are determined using valuation techniques that incorporate significant unobservable inputs. Depending on changes in the underlying assumptions and market conditions, the contingent forward and equity option instruments may be presented as either assets or liabilities in the accompanying condensed consolidated balance sheets.

The following table summarizes the valuation techniques and significant unobservable inputs used to estimate fair value:

Instrument	**Valuation Technique**	**Significant Unobservable Input**	**September 30, 2026**
Contingent forward instrument	Monte Carlo simulation	Expected volatility	50.0%
		Discount rate	4.2% - 13.1%
Royalty Notes	Scenario-based analysis	Discount rate	42.5% 11.9% - 12.7%
Derivative liability	Monte Carlo simulation	Expected volatility	70%
		Risk-free interest rate	4.0% - 5.0%
Equity option instrument	Monte Carlo simulation	Expected volatility	50.0%
		Discount rate	4.6% - 13.9%

Significant increases in expected future royalty cash flows would generally increase the fair value of the Royalty Notes, while increases in the discount rate would generally decrease its fair value. The fair values of the contingent forward instrument and option instrument are primarily affected by changes in expected share price volatility and,

where applicable, the probability of contingent settlement events. Changes in these assumptions may increase or decrease the fair values of the instruments and the related gains or losses recognized in earnings.

In addition to the financial instruments mentioned above, at September 30, 2026, the Company's financial instruments included cash and cash equivalents, restricted cash, accounts receivable, accounts receivable – related party, unbilled receivables – related party, unbilled receivables and accounts payable. The carrying amounts reported in the Company's condensed consolidated financial statements for these instruments approximate their respective fair values because of the short-term nature of these instruments.

Asset Retirement Obligations

Accounting for asset retirement obligations requires legal obligations associated with the retirement of long-lived assets to be recognized at fair value when incurred and capitalized as part of the related long-lived asset. In the absence of quoted market prices, the Company estimates the fair value of its asset retirement obligations using Level 3 present value techniques, in which estimates of future cash flows associated with retirement activities are discounted using a credit-adjusted risk-free rate. Asset retirement obligations currently reported on the condensed consolidated balance sheets were measured during a period of historically low interest rates. The impact on measurements of new asset retirement obligations using different rates in the future may be significant.

The Company uses estimates to determine the asset retirement obligations at the end of the lease term and discounts such asset retirement obligations using an estimated discount rate. Interest on the discounted asset retirement obligation is amortized over the term of the lease using the effective interest method and is recorded as financial related costs, net in the condensed consolidated statements of operations and comprehensive (loss) income.

The change in asset retirement obligations is as follows (in thousands):

	For the Nine-Month Periods Ended September 30,	
	2026	2025
Balance at beginning of period	$ 1,399	$ 2,821
Change in estimate	(371)	(1,476)
Amortization of interest	113	126
Effects of exchange rate changes	(12)	(106)
Balance at end of period	$ 1,129	$ 1,365

IDA Ireland Grant

In August 2021, MeiraGTx Ireland entered into an agreement pursuant to which it received a grant from IDA Ireland for financial assistance in establishing its operations in Shannon, Ireland. Under the terms of the grant, MeiraGTx Ireland is eligible to receive the lesser of €1.0 million or €10,000 for each job created (the "employment grant") and the lesser of €1.2 million or 4% of the actual expenditure on the provision of machinery and equipment (the "capital grant"). MeiraGTx Ireland may apply for a drawdown of the employment grant once a job has been created and the position has been held for a period of at least one month, and may apply for a drawdown of the capital grant once an eligible asset has been purchased and installed, conditioned on the creation of a cumulative number of jobs by the end of the immediately preceding year. An aggregate of 100 jobs must be created to receive the maximum benefit under the capital grant. An application for a drawdown must be accompanied by an audit certification for compliance with the terms of the grant. The Company has a guarantee in place with a bank in favor of IDA Ireland, pursuant to which it restricts cash in the amount of claims made under the grant such that the Company maintains the funds to cover any portion of the grant income that may become repayable in the future. This amount is presented as restricted cash in the accompanying condensed consolidated balance sheets. All expenditures were required to be completed by December 31, 2024, and the agreement terminates on the later of five years from the date of the last payment from the grant or five years from completion of the capital investment, which is expenditure of at least €30.0 million on eligible machinery and equipment.

The Company recognizes grant income when there is reasonable assurance that the Company will comply with the conditions attached to the grant and that it will receive the grant. During the nine months ended September 30, 2026 and 2025, the Company recognized de minimis grant income as a reduction of research and development expenses in the accompanying condensed consolidated statements of operations and comprehensive (loss) income.

Until the termination of the grant agreement in September 2030, MeiraGTx Ireland must maintain compliance with the terms of the grant. If the total number of jobs is less than 100 at the time of IDA Ireland's annual review, the Company may have to repay a portion of the capital grant, and if a job for which the Company received employment grant funding remains vacant for a period in excess of six calendar months, the Company may have to repay the employment grant received for that job. As of September 30, 2026, the Company is in compliance with the terms of the grant.

Revenue Recognition

The Company evaluates the promised goods or services to determine which promises, or group of promises, represent performance obligations. In contemplation of whether a promised good or service meets the criteria required of a performance obligation, the Company considers the stage of development of the underlying intellectual property, the capabilities and expertise of the customer relative to the underlying intellectual property, and whether the promised goods or services are integral to or dependent on other promises in the contract. When accounting for an arrangement that contains multiple performance obligations, the Company must develop judgmental assumptions, which may include market conditions, reimbursement rates for personnel costs, development timelines and probabilities of regulatory success to determine the stand-alone selling price for each performance obligation identified in the contract.

When the Company concludes that a contract should be accounted for as a combined performance obligation and recognized over time, the Company must then determine the period over which revenue should be recognized and the method by which to measure revenue. The Company generally recognizes revenue using a cost-based input method.

At inception, the Company determines whether contracts are within the scope of ASC 606 or other topics. For contracts that are determined to be within the scope of ASC 606, the Company recognizes revenue when its customer or collaborator obtains control of promised goods or services, in an amount that reflects the consideration which the Company expects to receive in exchange for those goods or services. To determine revenue recognition the Company performs the following five steps:

i. identify the contract(s) with a customer;

ii. identify the performance obligations in the contract;

iii. determine the transaction price;

iv. allocate the transaction price to the performance obligations within the contract; and

v. recognize revenue when (or as) the entity satisfies a performance obligation.

The Company only applies the five-step model to contracts when it determines that it is probable it will collect the consideration it is entitled to in exchange for the goods or services it transfers to the customer.

At contract inception, the Company assesses the goods or services promised within the contract to determine whether each promised good or service is a performance obligation. The promised goods or services in the Company's arrangements typically consist of a license to the Company's intellectual property and research, development and manufacturing services. The Company may provide options to additional items in such arrangements, which are accounted for as separate contracts when the customer elects to exercise such options, unless the option provides a material right to the customer. Performance obligations are promises in a contract to transfer a distinct good or service to the customer that (i) the customer can benefit from on its own or together with other readily available resources, and (ii) is separately identifiable from other promises in the contract. Goods or services that are not

individually distinct performance obligations are combined with other promised goods or services until such combined group of promises meet the requirements of a performance obligation.

The Company determines transaction price based on the amount of consideration the Company expects to receive for transferring the promised goods or services in the contract. Consideration may be fixed, variable, or a combination of both. At contract inception for arrangements that include variable consideration, the Company estimates the probability and extent of consideration it expects to receive under the contract utilizing either the most likely amount method or expected amount method, whichever best estimates the amount expected to be received. The Company then considers any constraints on the variable consideration and includes in the transaction price variable consideration to the extent it is deemed probable that a significant reversal in the amount of cumulative revenue recognized will not occur when the uncertainty associated with the variable consideration is subsequently resolved.

The Company then allocates the transaction price to each performance obligation based on the relative standalone selling price and recognizes as revenue the amount of the transaction price that is allocated to the respective performance obligation when (or as) control is transferred to the customer and the performance obligation is satisfied. For performance obligations which consist of licenses and other promises, the Company utilizes judgment to assess the nature of the combined performance obligation to determine whether the combined performance obligation is satisfied over time or at a point in time and, if over time, the appropriate method of measuring progress. The Company evaluates the measure of progress each reporting period and, if necessary, adjusts the measure of performance and related revenue recognition.

If there are multiple performance obligations, the Company allocates the transaction price to each performance obligation based on their estimated standalone selling prices ("SSP"). The Company estimates the SSP for each performance obligation by considering information such as market conditions, entity-specific factors, and information about its customer that is reasonably available. The Company considers estimation approaches that allow it to maximize the use of observable inputs. These estimation approaches may include the adjusted market assessment approach, the expected cost plus a margin approach or the residual approach. The Company also considers whether to use a different estimation approach or a combination of approaches to estimate the SSP for each performance obligation. Developing certain assumptions (e.g., treatable patient population, expected market share, probability of success and product profitability, and discount rate based on weighted-average cost of capital) to estimate the SSP of a performance obligation requires significant judgment.

The Company records amounts as contract assets when the right to consideration is deemed conditional. Contract assets are reclassified as accounts receivable once billed. When consideration is received, or such consideration is unconditionally due, from a customer prior to transferring goods or services to the customer under the terms of a contract, a contract liability is recorded as deferred revenue.

Amounts received prior to satisfying the revenue recognition criteria are recognized as deferred revenue in the Company's condensed consolidated balance sheets. Amounts expected to be recognized as revenue within the 12 months following the balance sheet date are classified as current deferred revenue. Amounts not expected to be recognized as revenue within the 12 months following the balance sheet date are classified as long-term deferred revenue.

The Company's revenue arrangements include the following:

Upfront License Fees: If a license is determined to be distinct from the other performance obligations identified in the arrangement, we recognize revenues from non-refundable, upfront fees allocated to the license when the license is transferred to the licensee and the licensee is able to use and benefit from the license. For licenses that are bundled with other promises, we utilize judgment to assess the nature of the combined performance obligation to determine whether the combined performance obligation is satisfied over time or at a point in time and, if over time, the appropriate method of measuring progress for purposes of recognizing revenue from non-refundable, upfront fees. We evaluate the measure of progress each reporting period and, if necessary, adjust the measure of performance and related revenue recognition.

Milestone Payments: At the inception of an agreement that includes research and development milestone payments, the Company evaluates each milestone to determine when and how much of the milestone to include in the transaction price. The Company first estimates the amount of the milestone payment that the Company could receive using either the expected value or the most likely amount approach. The Company primarily uses the most likely amount approach as that approach is generally most predictive for milestone payments with a binary outcome. Then, the Company considers whether any portion of that estimated amount is subject to the variable consideration constraint (that is, whether it is probable that a significant reversal of cumulative revenue would not occur upon resolution of the uncertainty). The Company updates the estimate of variable consideration included in the transaction price at each reporting date which includes updating the assessment of the likely amount of consideration and the application of the constraint to reflect current facts and circumstances.

Royalties: For arrangements that include sales-based royalties, including milestone payments based on a level of sales, and the license is deemed to be the predominant item to which the royalties relate, we will recognize revenue at the later of (i) when the related sales occur, or (ii) when the performance obligation to which some or all of the royalty has been allocated has been satisfied (or partially satisfied). To date, we have not recognized any revenue related to sales-based royalties or milestone payments based on the level of sales.

Research and Development Services: Under certain collaboration arrangements, the Company performs research and development services, including process development, process performance qualification, analytical development and other development activities. These services are generally transferred over time as the customer simultaneously receives and consumes the benefits of the Company's performance. Revenue is recognized over time using a cost-based input method that measures progress toward complete satisfaction of the performance obligation based on costs incurred relative to total estimated costs expected to satisfy the performance obligation.

Contract Manufacturing Services: Under certain collaboration and manufacturing arrangements, the Company provides contract manufacturing services, including manufacturing of product, release testing, stability testing, preparation of batch and assay documentation, and delivery of manufactured product. The Company evaluates the activities included in each request for proposal or work order to determine whether they represent separate performance obligations or should be combined. Activities are combined into a single performance obligation when they are not separately identifiable within the context of the contract and together represent a combined output to the customer. Revenue is recognized over time as the Company performs the manufacturing services because control of the work in process transfers to the customer as the manufacturing activities are performed. Progress toward complete satisfaction of the performance obligation is measured using a cost-based input method, under which revenue is recognized based on costs incurred relative to total estimated costs expected to satisfy the performance obligation.

Manufacturing Supply Services: Arrangements that include a promise for future supply of drug substance or drug product for either clinical development or commercial supply at the customer's discretion are generally considered options. The Company assesses if these options provide a material right to the licensee and if so, they are accounted for as separate performance obligations at the outset of the arrangement.

Customer Options: Customer options are evaluated at contract inception to determine whether those options provide a material right (i.e., an optional good or service offered for free or at a discount) to the customer. If the customer options represent a material right, the material right is treated as a separate performance obligation at the outset of the arrangement. The Company allocates the transaction price to material rights based on the standalone selling price. As a practical alternative to estimating the standalone selling price of a material right when the underlying goods or services are both (i) similar to the original goods or services in the contract and (ii) provided in accordance with the terms of the original contract, the Company allocates the total amount of consideration expected to be received from the customer to the total goods or services expected to be provided to the customer. Amounts allocated to any material right are recognized as revenue when or as the related future goods or services are transferred or when the option expires.

Research and Development

Research and development costs are charged to expense as incurred. These costs include, but are not limited to, employee-related expenses, including salaries, benefits and travel of the Company's research and development personnel; expenses incurred under agreements with contract research organizations and investigative sites that conduct clinical and preclinical studies and for the drug product for the clinical studies and preclinical activities; facilities; supplies; rent, insurance, certain legal fees, share-based compensation, depreciation and other costs associated with clinical and preclinical activities and regulatory operations. Research funding under collaboration agreements and refundable research and development credits / tax credits are recorded as an offset to these costs.

Costs for certain development activities, such as Company funded outside research programs, are recognized based on an evaluation of the progress to completion of specific tasks with respect to their actual costs incurred. Payments for these activities are based on the terms of the individual arrangements, which may differ from the pattern of costs incurred, and are reflected in the condensed consolidated financial statements as prepaid or accrued research and development expenses, as the case may be.

Treasury Shares

The Company accounts for treasury shares under the cost method. Treasury shares represent the Company's ordinary shares that have been issued and subsequently reacquired by the Company. The shares are recorded at cost as a reduction of shareholders' equity in the condensed consolidated balance sheets.

Treasury shares are held by the Company and may be reissued in the future; accordingly, they are not retired or cancelled upon repurchase.

Net (Loss) Income per Ordinary Share

Basic net (loss) income per ordinary share is computed by dividing net (loss) income attributable to ordinary shareholders by the weighted average number of the Company's ordinary shares assumed to be outstanding during the period of computation. Diluted net (loss) income per ordinary share is computed by dividing net (loss) income attributable to ordinary shareholders by the weighted average number of ordinary shares outstanding during the period, adjusted to give effect to potentially dilutive ordinary shares outstanding during the period using the treasury stock method. Potentially dilutive ordinary shares consist primarily of share options, restricted share units, and warrants. These securities are included in the computation of diluted net income per ordinary share when their effect is dilutive. Potentially dilutive securities are excluded from the computation of diluted earnings per ordinary share if their effect would be anti-dilutive. For periods in which the Company reports a net loss, all potentially dilutive ordinary shares are excluded from the computation of diluted net loss per ordinary share because their inclusion would be anti-dilutive. As a result, basic and diluted net loss per ordinary share are the same for such periods.

Segment Information

Operating segments are defined as components of an enterprise about which separate discrete information is available for evaluation by the chief operating decision maker ("CODM"), or decision-making group, in deciding how to allocate resources in assessing performance. The Company has one reportable and operating segment, which is the development and manufacturing of genetic medicines, for purposes of reporting financial condition and results of operations. The Company's CODM is the chief executive officer.

The accounting policies of its segment are the same as those described in the summary of significant accounting policies. The CODM allocates resources and assesses performance of the Company's single reportable segment by regularly reviewing the segment net (loss) income that also is reported on the condensed consolidated statements of operations and comprehensive (loss) income as net (loss) income.

The following table sets forth information about the Company's single reportable segment and the significant expenses reviewed by the CODM, including a reconciliation to net (loss) income (in thousands):

	For the Three-Month Periods Ended September 30,		For the Nine-Month Periods Ended September 30,	
	2026	2025	2026	2025
Service revenue - related party	$ 3,243	$ 410	$ 108,442	$ 6,027
Service revenue	1,571	—	13,456	—
License revenue - related party	—	—	204,643	—
Operating expenses:				
Cost of service revenue	617	—	2,018	—
Cost of service revenue - related party	2,806	313	8,767	4,367
General and administrative	7,016	9,751	22,328	24,699
Clinical programs:				
Botaretigene sparoparvovec	2,321	—	33,655	—
AAV-hAQP1	6,148	1,997	15,852	9,603
AAV-GAD	4,555	1,200	8,565	11,630
Other ocular diseases	—	2,915	108	4,380
Manufacturing	12,412	12,568	33,821	35,557
Preclinical programs:				
Gene regulation	2,440	2,683	5,112	8,210
Neurodegenerative diseases	292	271	1,020	995
Ocular diseases	23	688	1,026	2,475
Other research and development[1]	5,554	4,968	16,763	10,620
Share-based compensation	7,663	5,952	15,096	16,504
Depreciation and amortization	2,845	3,155	8,680	9,427
Total operating expenses	54,692	46,461	172,811	138,467
Other segment items[2]	(1,403)	(4,462)	(90,655)	3,151
Segment net (loss) income	$ (51,281)	$ (50,513)	$ 63,075	(129,289)
Net (loss) income	$ (51,281)	$ (50,513)	$ 63,075	$ (129,289)

[1] Other research and development is comprised of all other costs including payroll and payroll related costs, travel, rent and facilities costs and other non-program specific expenses.

[2] Other segment items is comprised of foreign currency (loss) gain, interest income, financial related costs, net, other income (expense), loss on equity method investee and income tax expense.

The Company's service revenue – related party and service revenue were generated in the United Kingdom and the Company's license revenue – related party was generated in the United States.

The following table summarizes long-lived assets by geographical area (in thousands):

	September 30, 2026	December 31, 2025
United States	$ 7,930	$ 8,830
United Kingdom	23,835	26,092
European Union	98,112	107,589
	$ 129,877	$ 142,511

Non-Controlling Interests

Non-controlling interests are classified as a separate component of equity in the condensed consolidated balance sheets and condensed consolidated statement of shareholders' equity. Additionally, net (loss) income and comprehensive (loss) income attributable to non-controlling interests are reflected separately from net (loss) income and comprehensive (loss) income on the condensed consolidated statements of operations and comprehensive (loss) income and condensed consolidated statement of shareholders' equity. Any change in ownership of a subsidiary while the controlling financial interest is retained is accounted for as an equity transaction between the controlling and non-controlling interests. Losses continue to be attributed to the non-controlling interests, even when the non-controlling interests' basis has been reduced to zero.

Accounting Pronouncements Adopted

Effective January 1, 2026, the Company adopted ASU 2025-05, *Financial Instrument – Credit Losses (ASC 326)*: *Measurement of Credit Losses for Accounts Receivable and Contract Assets*, which provides targeted improvements to the accounting for credit losses. The adoption of this standard did not have a material impact on the Company's condensed consolidated financial statements or related disclosures.

In September 2025, the FASB issued ASU 2025-07, *Derivatives and Hedging (ASC 815) and Revenue from Contracts with Customers (ASC 606): Derivative Scope Refinements and Scope Clarification for Share-Based Noncash Consideration from a Customer in a Revenue Contract*, which refines the scope of ASC 815 by clarifying which contracts are subject to derivative accounting and expands the scope exception for certain contracts not traded on an exchange to include contracts for which settlement is based on operations or activities specific to one of the parties to the contract. The guidance also provides clarification under ASC 606 for share-based payments from a customer in a revenue contract. The adoption of this standard did not have a material impact on the Company's condensed consolidated financial statements or related disclosures.

Recent Accounting Pronouncements Not Yet Adopted

In November 2024, the FASB issued ASU 2024-03, *Income Statement: Reporting Comprehensive Income: Expense Disaggregation Disclosures (Subtopic 220-40)*, which requires public business entities to disclose additional information about specific expense categories in the notes to financial statements at interim and annual reporting periods. In January 2025, the FASB issued ASU 2025-01, *Clarifying the Effective Date*, which revised the effective date of ASU 2024-03 for interim periods. The guidance is effective for annual periods beginning after December 15, 2026, and interim periods within fiscal years beginning after December 15, 2027. The Company is currently assessing the impact of ASU 2024-03 and ASU 2025-01 on its condensed consolidated financial statements.

In December 2025, the FASB issued ASU 2025-10, *Government Grants (ASC 832): Accounting for Government Grants Received by Business Entities*. This update establishes guidance on the recognition, measurement, and presentation of government grants received by business entities. The new guidance leverages the principles in the accounting framework for government assistance in International Accounting Standard 20, *Accounting for Government Grants and Disclosure of Government Assistance*. The guidance is effective for interim periods within annual reporting periods beginning after December 15, 2028. Early adoption is permitted. The Company is currently evaluating the impact of ASU 2025-10 on its condensed consolidated financial statements and related disclosures.

In December 2025, the FASB issued ASU 2025-11, *Interim Reporting (ASC 270) Narrow-Scope Improvements*. This update clarifies interim disclosure requirements, including providing a comprehensive list of interim disclosure requirements under GAAP and a disclosure principle that requires entities to disclose events since the last annual reporting period that have a material impact on the entity. The guidance is effective for interim periods within annual reporting periods beginning after December 15, 2027. Early adoption is permitted. The Company does not expect any significant impact on its financial condition or results of operations upon adoption.

In December 2025, the FASB issued ASU 2025-12, *Codification Improvements*, to update, correct, clarify and otherwise improve GAAP. The guidance is effective for annual reporting periods beginning after December 15, 2026, and for interim periods within those annual reporting periods. Early adoption is permitted as of the beginning of an annual reporting period and adoption can be applied prospectively or retrospectively, as well as on an issue-by-issue basis. The Company does not expect any significant impact on its financial condition or results of operations upon adoption.

3. Equity Method and Other Investments

The Company's investments consist of the following (in thousands):

		September 30, 2026		
Investee	**Investment Type**	**Ownership Percentage**	**Carrying Value**	**Cost Basis**
Reogen	Equity Method Investment	39.0 %	$ 22,440	$ 98,502
Hologen Limited	Equity Investment	3.0 %	6,810	6,810
Visiogene LLC	Equity Method Investment	25.0 %	5,133	5,165
Other	Equity Investment	0.8 %	1,616	1,500
Total equity method and other investments			$ 35,999	$ 111,977

		December 31, 2025		
Investee	**Investment Type**	**Ownership Percentage**	**Carrying Value**	**Cost Basis**
Visiogene LLC	Equity Method Investment	25.0 %	$ 5,133	$ 5,165
Other	Equity Investment	0.8 %	1,616	1,500
Total equity method and other investments			$ 6,749	$ 6,665

On April 20, 2026, the Company completed the initial closing of its strategic collaboration with Hologen and recognized a 39% equity interest in Reogen. The Company accounts for its investment in Reogen under the equity method of accounting because it has the ability to exercise significant influence over Reogen's operating and financial policies but does not control the entity. As part of the accounting for the strategic collaboration arrangement, the initial carrying amount of the investment was measured based on the fair value of the underlying 39% equity interest acquired. Thereafter, the carrying amount of the investment is adjusted to recognize the Company's proportionate share of Reogen's net income or loss and other comprehensive income or loss. As of September 30, 2026, the carrying amount of the Company's investment in Reogen was $22.4 million. During the three and nine months ended September 30, 2026, the Company recognized a loss of $3.9 million and $75.8 million, respectively, under the equity method of accounting, representing its proportionate share of Reogen's operating results associated with the acquired in-process research and development assets relating to the Clinical Programs and the Delivery Device, as well as Reogen's ongoing research and development activities.

Below is the summarized financial information of Reogen for the three months ended September 30, 2026 and period between April 20, 2026 and September 30, 2026 (in thousands):

	Three-Month Period Ended September 30, 2026	For the period between April 20, 2026 and September 30, 2026
Statement of Operations		
Research and development expense	$ (10,064)	$ (194,445)
General and administrative expense	$ (40)	$ (60)
Other income	$ 52	$ 95
Net loss	$ (10,052)	$ (194,410)

In connection with the strategic collaboration, the Company acquired an aggregate of 500,000 ordinary shares of Hologen, representing an ownership interest of approximately 3%. The investment is accounted for as an equity investment in accordance with ASC 321, *Investments – Equity Securities*. As part of the accounting for the strategic collaboration arrangement, the investment was initially recognized based on the fair value assigned to the underlying equity interest acquired. Because Hologen is a privately held company without a readily determinable fair value and has multiple classes of shares with differing economic rights and preferences, the fair value assigned to the Company's investment reflects the rights and characteristics of the specific ordinary shares acquired. Subsequently, the investment is measured using the measurement alternative under ASC 321, at cost, less impairment, if any, adjusted for observable price changes in orderly transactions for an identical or similar investment of the same issuer. As of September 30, 2026, the carrying amount of the Company's investment in Hologen was $6.8 million. Accordingly, the carrying amount of the investment should not be used to infer the overall equity value of Hologen or the value of other classes of Hologen shares.

4. Accrued Expenses

Accrued expenses for the periods presented are comprised of the following (in thousands):

	September 30, 2026	December 31, 2025
Research and development	$ 5,233	$ 3,867
Professional fees	3,629	6,069
Compensation and benefits	3,580	12,810
Manufacturing costs	2,162	3,378
Consulting	1,317	1,041
Clinical trial costs	1,212	1,429
Interest on Perceptive Notes	—	2,710
Other	350	1,589
	$ 17,483	$ 32,893

5. Financial Instruments

The Company recognizes certain financial instruments arising from financing and strategic collaboration arrangements. These instruments are measured either at fair value on a recurring basis or in accordance with their applicable accounting guidance. Depending on changes in fair value, these instruments may be presented as either assets or liabilities in the accompanying condensed consolidated balance sheets.

Financial instruments for the periods presented are comprised of the following (in thousands):

Financial Instruments	September 30, 2026	December 31, 2025
Contingent forward instrument	$ 21,000	$ —
Derivative liability	$ (4,433)	$ —
Equity option instrument	$ (2,085)	$ —

As described in Note 1, the Company recognized a contingent forward instrument related to its obligation to issue additional MeiraGTx Manufacturing shares upon Hologen's payment of the additional funding commitment, and an option instrument related to Hologen's contractual right to increase its ownership interest in MeiraGTx Manufacturing. These instruments are measured at fair value on a recurring basis using a Monte Carlo simulation model and are classified within Level 3 of the fair value hierarchy due to the use of significant unobservable inputs. Changes in fair value are recognized in earnings in the period in which they occur. As of September 30, 2026, the contingent forward instrument and option instrument were presented within other current assets and other current liabilities, respectively, in the accompanying condensed consolidated balance sheets based on their respective fair values at the reporting date. During the three and nine months ended September 30, 2026, the Company recognized a loss of $0.8 million change in fair value of contingent forward instrument in other income (expense) of the condensed consolidated statements of operations and comprehensive (loss) income. During the three and nine months ended September 30, 2026, the Company recognized a gain of $0.4 million change in fair value of equity option instrument in other income (expense) of the condensed consolidated statements of operations and comprehensive (loss) income.

The Company also has a derivative liability associated with the Right and Offering Participation granted under the Securities Purchase Agreement entered into with the Purchasers (as such capitalized terms are defined in, and such transactions are described in, Note 12). The instrument is classified as a liability because it may require settlement through the issuance of a variable number of the Company's ordinary shares for a fixed monetary amount and is accounted for at fair value on a recurring basis. The derivative liability is classified within Level 3 of the fair value hierarchy as its valuation incorporates significant unobservable inputs, and changes in fair value are recognized in earnings in the period in which they occur. As of September 30, 2026, the derivative liability was presented within other current liabilities in the accompanying condensed consolidated balance sheets based on their respective fair values at the reporting date. In the second quarter of 2026, the Company recognized a loss of $5.2 million on the initial measurement of derivative liability in other income (expense) of the condensed consolidated statements of operations and comprehensive (loss) income. During the three and nine months ended September 30, 2026, the Company recognized a gain of $0.8 million change in fair value of derivative liability in other income (expense) of the condensed consolidated statements of operations and comprehensive (loss) income, which is included in non-cash other income on the condensed consolidated statements of cash flows.

6. Share-Based Compensation

Equity Incentive Plans

The Company's 2018 Incentive Award Plan (the "2018 Plan") and 2016 Equity Incentive Plan (the "2016 Plan") were adopted by the Company's board of directors and shareholders. Under the 2018 Plan and the 2016 Plan, the Company has granted share options and restricted share units ("RSUs") to selected officers, employees, non-employee members of the board of directors and non-employee consultants. Upon the adoption of the 2018 Plan, the Company ceased issuing awards under the 2016 Plan.

In June 2026, the Board approved the 2026 Employment Inducement Award Plan (the "2026 Plan", and together with the 2018 Plan and the 2016 Plan, the "Plans") to make equity awards to newly hired employees as a material inducement to employment in accordance with Nasdaq Rule 5635(c)(4).

The Company's board of directors or a committee thereof administers the Plans.

Options

A summary of the Company's share option activity related to employees, non-employee members of the board of directors and non-employee consultants as of December 31, 2025 and for the nine-month period ended September 30, 2026 is as follows (in thousands, except share and per share amounts):

	Number of Options	Weighted-Average Exercise Price	Weighted-Average Remaining Contractual Term (years)
Outstanding at December 31, 2025	7,859,338	$ 12.29	4.58
Granted	409,150	$ 11.30	
Exercised	(215,622)	$ 6.70	
Forfeited	(366,856)	$ 14.65	
Expired	(198,197)	$ 7.72	
Outstanding at September 30, 2026	7,487,813	$ 12.52	4.39
Options exercisable at September 30, 2026	6,752,840	$ 12.86	3.93
Options vested and expected to vest at September 30, 2026	7,487,813	$ 12.52	4.39
Aggregate intrinsic value of options outstanding as of September 30, 2026	$ 12,366		
Aggregate intrinsic value of options exercisable as of September 30, 2026	$ 11,061		

Options granted under the Plans have a maximum contractual term of ten years. Options granted generally vest 25% on the first anniversary of the date of grant and the balance ratably over the next 36 months. Annual options granted to directors generally vest on the earlier of the first anniversary of the date of grant or the day before the Company's annual meeting of shareholders after the date of grant.

The Company recorded the following share-based compensation expense in connection with the options for the three-month and nine-month periods ended September 30, 2026 and 2025 (in thousands):

	Three-Month Periods Ended September 30,	
	2026	2025
Research and development	$ 1,677	$ 857
General and administrative	279	472
Total share-based compensation	$ 1,956	$ 1,329

	Nine-Month Periods Ended September 30,	
	2026	2025
Research and development	$ 2,325	$ 2,639
General and administrative	833	1,569
Total share-based compensation	$ 3,158	$ 4,208

The total fair value of options vested during the three-month periods ended September 30, 2026 and 2025 was $0.5 million and $1.3 million, respectively.

The total fair value of options vested during the nine-month periods ended September 30, 2026 and 2025 was $2.1 million and $4.7 million, respectively.

The weighted-average grant date fair value of options granted during the nine-month periods ended September 30, 2026 and 2025 was $7.06 and $3.85 per share, respectively.

The grant date fair values of the share options granted were estimated using the Black-Scholes option valuation model with the following ranges of assumptions:

	2026	2025
Risk-free interest rate	4.20 - 4.43%	3.81 - 4.13%
Expected volatility	64%	67%
Expected dividend yield	0%	0%
Expected term (in years)	6.01	6.01

As of September 30 2026, the total compensation expense relating to unvested options granted that had not yet been recognized was $4.1 million, which is expected to be realized over a period of 3.8 years. The Company will issue shares upon exercise of options from ordinary shares reserved under the Plans.

Restricted Share Units

A summary of the Company's RSU activity related to employees, non-employee members of the board of directors and non-employee consultants as of December 31, 2025 and for the nine-month period ended September 30, 2026 is as follows:

	Number of Restricted Share Units		Weighted-Average Grant Date Fair Value
Outstanding at December 31, 2025	6,184,250	$	7.00
Granted	3,584,244	$	11.28
Vested	(1,939,250)	$	8.60
Forfeited	(65,500)	$	6.62
Outstanding at September 30, 2026	7,763,744	$	8.58

RSUs granted under the Plans generally vest 50% on the second anniversary of the date of grant and 25% on the third and fourth anniversaries of the date of grant. RSUs granted to directors when they join the board generally vest in three equal annual installments on each of the first, second, and third anniversaries of the grant date, and annual RSUs granted to directors generally vest in a single installment on the earliest to occur of the first anniversary of the grant date or the day immediately prior to the date of the next annual meeting of the Company's shareholders occurring after the date of grant. The RSUs granted to the directors in June 2021 will be paid on or within 30 days after the date a director ceases to serve on the board. For RSUs granted in June 2022 and future years, the directors may annually elect whether to defer the payment of their annual RSU awards under the Deferred Compensation Plan for Non-Employee Directors, which was adopted by the board on December 17, 2021. As of September 30, 2026, there were 722,500 vested shares that have been deferred and are excluded from ordinary shares outstanding. The related share-based compensation expense, which is recognized ratably over the requisite service period, is included in general and administrative and research and development expenses, as applicable, in the condensed consolidated statements of operations and comprehensive (loss) income.

The Company recorded the following share-based compensation expense in connection with the RSUs for the three-month and nine-month periods ended September 30, 2026 and 2025 (in thousands):

	Three-Month Periods Ended September 30,	
	2026	2025
Research and development	$ 1,093	$ 1,367
General and administrative	4,614	3,256
Total share-based compensation	$ 5,707	$ 4,623

	Nine-Month Periods Ended September 30,	
	2026	2025
Research and development	$ 2,373	$ 3,723
General and administrative	9,565	8,573
Total share-based compensation	$ 11,938	$ 12,296

As of September 30, 2026, the total compensation expense relating to unvested RSUs granted that had not yet been recognized was $52.9 million, which is expected to be realized over a period of 3.8 years.

To satisfy employee minimum statutory tax withholding requirements for restricted share units that vest, the Company withholds a portion of the vested ordinary shares. During the nine months ended September 30, 2026 and 2025, the Company withheld 798,155 and 433,960 ordinary shares with a total value of $6.0 million and $3.0 million, respectively. These amounts are presented as a cash outflow from financing activities in the accompanying condensed consolidated statements of cash flows.

During the three-month and nine-month periods ended September 30, 2026 and 2025, the Company recognized total share-based compensation expense in the accompanying condensed consolidated statements of operations and comprehensive (loss) income as follows (in thousands):

	Three-Month Periods Ended September 30,	
	2026	2025
Research and development	$ 2,770	$ 2,224
General and administrative	4,893	3,728
Total share-based compensation	$ 7,663	$ 5,952

	Nine-Month Periods Ended September 30,	
	2026	2025
Research and development	$ 4,698	$ 6,362
General and administrative	10,398	10,142
Total share-based compensation	$ 15,096	$ 16,504

The Company does not expect to realize any tax benefits from its share option activity or the recognition of share-based compensation expense because it maintains a full valuation allowance against its deferred tax assets. While the Company may report taxable income in certain periods as a result of non-recurring transactions, management has concluded that such income is not sufficient positive evidence to overcome the significant negative evidence supporting the valuation allowance. Accordingly, no amounts related to excess tax benefits have been reported in cash flows from operations or cash flows from financing activities for the nine-month periods ended September 30, 2026 and 2025.

7. Ordinary Shares

At-the-Market

In December 2023, the Company entered into an "at-the-market" sales agreement with BofA Securities, Inc., or BofA, pursuant to which the Company may sell from time to time, ordinary shares having an aggregate offering price of up to $100.0 million through BofA, acting as the Company's agent. During the nine-month periods ended September 30, 2026 and 2025, the Company raised gross proceeds of $60.8 million and $9.9 million, respectively, through the sale of 5,574,968 and 1,510,300 ordinary shares, respectively, pursuant to an "at-the-market" equity offering program. Under the "at-the-market" equity program which is currently effective and may remain available for the Company to use in the future, the Company may sell up to an additional $15.9 million of ordinary shares. Whether the Company chooses to affect future sales under the "at-the-market" equity offering program will depend on a number of factors, including, among others, market conditions and the trading price of the Company's ordinary shares relative to other sources of capital.

Equity Financing

On April 17, 2026, the Company completed a registered offering of 11,111,111 ordinary shares at a price of $9.00 per share, resulting in gross proceeds of approximately $100.0 million and net proceeds of $93.2 million, after deducting underwriting discounts, commissions and offering expenses payable by the Company.

Treasury Shares

On December 31, 2025, the Company entered into a share purchase agreement to repurchase 2,300,000 of its ordinary shares from Perceptive Life Sciences Master Fund, Ltd., an affiliate of Perceptive Advisors LLC, at a purchase price of $7.91 per share, for an aggregate purchase price of $18.2 million. The repurchase transaction was completed on January 5, 2026, at which time the Company acquired the shares and recorded them as treasury shares, resulting in a reduction of shareholders' equity.

8. Earnings Per Share

	Three-Month Periods Ended September 30,		Nine-Month Periods Ended September 30	
	2026	2025	2026	2025
Net income attributable to ordinary shareholders (numerator, in thousands)	$ (51,212)	$ (50,513)	$ 63,195	$ (129,289
Weighted average ordinary shares outstanding – basic	96,879,809	80,947,703	89,923,492	80,195,572
Dilutive effect of:				
Share options	—	—	797,854	—
Restricted share units	—	—	2,589,884	—
Warrants	—	—	134,899	—
Weighted average ordinary shares outstanding – diluted	96,879,809	80,947,703	93,446,129	80,195,572
Basic earnings per ordinary share	$ (0.53)	$ (0.62)	$ 0.70	$ (1.61
Diluted earnings per ordinary share	$ (0.53)	$ (0.62)	$ 0.68	$ (1.61

The following securities are considered to be ordinary share equivalents, but were not included in the computation of diluted net loss per ordinary share for the three and nine-month periods ended September 30, 2026 and 2025 because their effect was anti-dilutive:

	Three-Month Periods Ended September 30,		Nine-Month Periods Ended September 30,	
	2026	2025	2026	2025
Share options	7,487,813	7,964,365	3,297,830	7,964,365
Restricted share units	7,763,744	6,182,250	—	6,182,250
Warrants	700,000	700,000	—	700,000
	15,951,557	14,846,615	3,297,830	14,846,615

9. Income Taxes

The Company recorded an income tax provision for the three and nine months ended September 30, 2026, primarily related to income generated in the United States and in the United Kingdom as a result of certain non-recurring transactions completed during the period. The Company's estimated annual effective tax rate excludes jurisdictions for which a reliable estimate cannot be made due to anticipated losses for which no tax benefit is expected to be recognized.

The Company periodically evaluates the realizability of its deferred tax assets based on all available positive and negative evidence. The realization of deferred tax assets depends on the Company's ability to generate sufficient future taxable income prior to the expiration of the underlying tax attributes. The Company recognized income in the United States and in the United Kingdom during the current period; however, the Company concluded that such income was primarily attributable to non-recurring transactions and does not constitute sufficient positive evidence to support the realization of its deferred taxes. Accordingly, the Company continues to maintain a full valuation allowance against its deferred tax assets (after consideration of deferred tax liabilities related to right-of-use assets and fixed assets) in the United States, United Kingdom, Ireland, France and the Netherlands as of September 30, 2026.

Should the Company determine that it is more likely than not that some or all of its deferred tax assets will be realized in the future, the valuation allowance would be reduced in the period such determination is made, resulting in the recognition of a corresponding income tax benefit.

10. Related-Party Transactions

Relationship with Janssen Pharmaceuticals, Inc.

On December 20, 2023, the Company entered into the Original Asset Purchase Agreement and Supply Agreement with Janssen. The agreements were combined and accounted for as a single contract under ASC 606 because they were negotiated with a single commercial objective. The transaction price was allocated to the identified performance obligations, and revenue was recognized as those performance obligations were satisfied.

On April 15, 2026, the Company entered into the Asset Purchase Agreement with Janssen to reacquire all rights, title and interest in bota-vec and other assets related to the RPGR Product. In connection with this transaction, the Company made an upfront cash payment of $25.0 million to Janssen, and agreed to make certain future milestone and royalty payments tied to regulatory approval and commercial performance. Concurrently, the parties entered into the Termination Agreement that terminated the Original Asset Purchase Agreement, the Supply Agreement and certain other documents related to the Original Asset Purchase Agreement. As a result of the termination, the Company had no remaining performance obligations under the foregoing agreements.

The Company accounted for the reacquisition of bota-vec as an asset acquisition. The total consideration transferred under the Asset Purchase Agreement included a $25.0 million upfront payment and the settlement of the outstanding

$3.3 million related party accounts receivable. The consideration was allocated to the reacquired in-process research and development assets, inventory acquired from Janssen and the Transition Services Agreement between MeiraGTx Ocular and Janssen, dated as of the Janssen Closing Date (the "Transition Services Agreement"), based on their relative fair values. Because the reacquired in-process research and development assets had no alternative future use, the portion of the consideration allocated to those assets was recognized as acquired in-process research and development expense during the second quarter of 2026. The portion of the consideration allocated to the acquired inventory was recognized as research and development expense as the inventory has no alternative future use and will be consumed in the Company's internal research and development activities. The portion of the upfront payment allocated to the Transition Services Agreement is recognized as expense as the related services are performed.

Accordingly, during the three and nine months ended September 30, 2026, the Company recognized the remaining deferred revenue associated with the Original Asset Purchase Agreement and related agreements as service revenue – related party, as the remaining performance obligations were extinguished upon termination of the agreements.

A summary of the deferred revenue – related party is as follows (in thousands):

Deferred revenue at December 31, 2025	$ 66,896
Other amounts collected or invoiced	65
Deferred revenue recognized as service revenue during the three-month period ended March 31, 2026	(125)
Deferred revenue at March 31, 2026	66,836
Recognized service revenue due to termination of agreement during the three-month period ended June 30, 2026	(66,836)
Deferred revenue at June 30, 2026	$ —
Deferred revenue at September 30, 2026	$ —

During the nine-month period ended September 30, 2026, the Company recognized related party service revenue of $67.0 million, of which $66.8 million related to the recognition of related party deferred revenue following the termination of the Original Asset Purchase Agreement and related agreements and $0.1 million related to the recognition of related party deferred revenue based on cumulative progress of PPQ services under the Original Asset Purchase Agreement and related agreements. During the nine-month period ended September 30, 2025, the Company recognized $6.0 million of related party service revenue, inclusive of the $2.3 million of related party deferred revenue recognized as related party service revenue, based on cumulative progress of PPQ services under the Original Asset Purchase Agreement and related agreements.

Relationship with Hologen

During the second quarter of 2026, the Company and Hologen and their affiliates completed the initial closing of the strategic collaboration and entered into amendments to the framework agreements to facilitate the initial funding and investment structure. As part of the initial closing, the parties entered into the Reogen Collaboration Agreement for the development, manufacturing and commercialization of the Clinical Programs and the Delivery Device, completed Hologen's initial investment in MeiraGTx Manufacturing, and agreed to the related equity arrangements providing for Hologen's potential acquisition of additional equity interests in MeiraGTx Manufacturing in the future.

The agreements entered into with Hologen and its affiliates were negotiated as interdependent elements of the overall strategic collaboration and were intended to achieve a single commercial objective. Although executed on different dates, the agreements were evaluated collectively based on their interrelationship and economic substance. Accordingly, the agreements were combined and accounted for as a single contract. As part of the accounting for the combined contract, the Company first identified the financial assets, financial liabilities and equity transactions arising from the agreements, including its investments in Reogen and Hologen, its sale of a non-controlling interest in MeiraGTx Manufacturing to Hologen, and the related equity contracts, and accounted for those elements at fair value. The residual consideration attributable to the revenue contract, including cash and non-cash consideration, was then accounted for in accordance with ASC 606. The resulting transaction price was allocated to the identified

performance obligations, including the transfer of the licenses to the Clinical Programs and the Delivery Device, development services, transition services and other contractual obligations, based on their relative standalone selling prices. Revenue has been recognized as the related performance obligations are satisfied. As of September 30, 2026, the aggregate transaction price allocated to unsatisfied performance obligations was $15.9 million.

During the three months ended September 30, 2026, the Company and its affiliates entered into the Device Assignment Agreement with Reogen, a related party, and its affiliates, pursuant to which the Company assigned certain intellectual property relating to, and contractual rights associated with the development of, the Delivery Device to Reogen, and amended the Reogen Collaboration Agreement to reflect the foregoing.

In connection with the contractual share repurchase provisions under the Reogen collaboration arrangements, the Company also recognized a refund liability of $7.3 million representing its obligation to transfer a specified number of Reogen shares to Hologen. In accordance with ASC 606, the refund liability was initially measured based on the fair value of the specified number of Reogen shares at the transaction date and is presented within other current liabilities in the accompanying condensed consolidated balance sheets. The refund liability will be derecognized upon settlement of the contractual share transfer.

As part of the arrangement, the Company recognized license revenue related to the transfer of the licenses to the Clinical Programs and the Delivery Device in accordance with its accounting policy. In addition, a portion of the transaction consideration was allocated to future development services and transition services associated with the Clinical Programs and the Delivery Device. Accordingly, the Company recorded related party deferred revenue representing the unsatisfied performance obligations for the development and transition services, which will be recognized as the related performance obligations are satisfied over the expected service period.

During the nine months ended September 30, 2026, the Company recognized license revenue – related party of $204.6 million related to the licenses of the Clinical Programs and the Delivery Device. The Company recognized $3.2 million and $41.1 million of service revenue – related party associated with development and transition services provided to Reogen for the three and nine months ended September 30, 2026, respectively. As of September 30, 2026, the Company has invoiced Reogen for $23.1 million for the development and transition services and recognized a related party unbilled receivable of $17.2 million, representing revenue recognized in excess of amounts billed under the Reogen Collaboration Agreement. As of September 30, 2026, the remaining deferred revenue balance related to the Reogen Collaboration Agreement was $15.9 million, with $4.7 million of this balance expected to be recognized as revenue within the next 12 months as the related services are performed, with the remainder expected to be recognized in two to three years as the remaining performance obligations are satisfied.

A summary of the deferred revenue – related party is as follows (in thousands):

Deferred revenue at December 31, 2025	$	—
Initial recognition upon execution of the Reogen Collaboration Agreement		16,726
Revenue recognized as service revenue during the period		(549)
Deferred revenue at June 30, 2026		16,177
Revenue recognized as service revenue during the period		(355)
Effects of foreign exchange		55
Deferred revenue at September 30, 2026	$	15,877

Debt Financing

On August 2, 2022 the Company, as borrower, and MeiraGTx UK II and MeiraGTx Ireland, as guarantors (the "Subsidiary Guarantors"), entered into a senior secured financing arrangement (the "Financing Agreement") by and among the Company, the Subsidiary Guarantors, the lenders and other parties from time to time party thereto and Perceptive Credit Holdings III, LP, as administrative agent and lender ("Perceptive"). On December 19, 2022, the Financing Agreement was converted to a notes purchase agreement and guaranty (the "Notes Purchase Agreement") between the same parties and under substantially the same terms and conditions as the Financing Agreement, subject to certain customary note constitution terms. Pursuant to an amendment to the Notes Purchase Agreement entered

into on March 25, 2026, the maturity date of the Notes Purchase Agreement was extended from August 2, 2026 to May 2, 2027 and the Company agreed to redeem a portion of the outstanding principal amount equal to $25.0 million on or before June 30, 2026. The parties also agreed to amend the warrants further described in Note 12 to change the exercise price to $8.00 per share. Pursuant to another amendment to the Notes Purchase Agreement entered into on May 12, 2026, the maturity date of the Notes Purchase Agreement was extended from May 2, 2027 to July 1, 2027. In the second quarter of 2026, the Company voluntarily prepaid all outstanding principal and accrued interest under the Notes Purchase Agreement, resulting in the full repayment and termination of such agreement. In connection with the repayment, the Company recognized a loss on extinguishment of debt of $0.3 million related to the write-off of unamortized debt issuance costs. Refer to the discussion in Note 12 for further information related to the accounting for the debt financing. Perceptive Advisors LLC, an affiliate of Perceptive, is a greater than 10% holder of the ordinary shares of the Company. Additionally, Ellen Hukkelhoven, Ph.D., a director of the Company, is an employee of Perceptive Advisors LLC.

Share Repurchase

On December 31, 2025, the Company entered into a share purchase agreement to repurchase 2,300,000 of its ordinary shares from Perceptive Life Sciences Master Fund, Ltd., an affiliate of Perceptive Advisors LLC, at a purchase price of $7.91 per share, for an aggregate purchase price of $18.2 million. The repurchase transaction was completed on January 5, 2026, at which time the Company acquired the shares and recorded them as treasury shares, resulting in a reduction of shareholders' equity.

11. Leases

The Company has commitments under operating leases for laboratory, warehouse, clinical trial sites and office space. The Company also has finance leases for manufacturing space and office equipment. The Company's leases have initial lease terms ranging from 1.5 years to 191 years. Certain lease agreements contain provisions for future rent increases. Payments due under the lease contracts include fixed payments.

Total rent expense under these leases was $0.9 million and $1.3 million for the three-month periods ended September 30, 2026 and 2025, respectively.

Total rent expense under these leases was $3.0 million and $3.7 million for the nine-month periods ended September 30, 2026 and 2025, respectively.

There were no new leases recognized during the nine-month period ended September 30, 2026. The Company entered into a lease agreement for laboratory space in March 2025 with an initial lease term of 91 months. The Company also entered into a lease agreement for office space in July 2025 with an initial lease term of 39 months. The lease for the laboratory space contains provisions for tenant allowances and future rent increases. The Company took possession of both spaces in August 2025 and recognized initial operating lease right-of-use assets in the amount of $8.2 million and corresponding lease liabilities in the amount of $8.0 million, in connection with these leases, which are included in the right-of-use assets and lease obligations in the condensed consolidated balance sheets as of September 30, 2025. During the nine months ended September 30, 2025, the Company determined that it was reasonably certain to exercise its early termination option on an operating lease for laboratory and office space,

resulting in a remeasurement of the right-of-use asset and lease liability reflected in the accompanying condensed consolidated balance sheets.

The components of lease cost for the three-month and nine-month periods ended September 30, 2026 and 2025 are as follows (in thousands):

	Three-Month Periods Ended September 30,	
	2026	**2025**
Finance lease cost		
Amortization of right-of-use assets	$ 311	$ 304
Interest on lease liabilities	4	—
Total finance lease cost	315	304
Operating lease cost	1,018	1,276
Short-term lease cost	203	103
Total lease cost	$ 1,536	$ 1,683

	Nine-Month Periods Ended September 30,	
	2026	**2025**
Finance lease cost		
Amortization of right-of-use assets	$ 931	$ 891
Interest on lease liabilities	14	—
Total finance lease cost	945	891
Operating lease cost	3,204	3,673
Short-term lease cost	514	298
Total lease cost	$ 4,663	$ 4,862

Amounts reported in the condensed consolidated balance sheets for leases where the Company is the lessee as of September 30, 2026 and December 31, 2025 were as follows (in thousands):

	September 30, 2026	**December 31, 2025**
Operating leases		
Right-of-use assets	$ 10,913	$ 12,852
Capitalized lease obligations	$ 11,836	$ 14,202
Finance leases		
Right-of-use assets	$ 22,076	$ 23,616
Capitalized lease obligations	$ 115	$ 147
Weighted-average remaining lease term		
Operating leases	6.0 years	6.4 years
Finance leases	172.2 years	172.9 years
Weighted-average discount rate		
Operating leases	10.4 %	11.2 %
Finance leases	13.0 %	13.0 %

Other information related to leases for the three-month and nine-month periods ended September 30, 2026 and 2025 are as follows (in thousands):

	Three-Month Periods Ended September 30,	
	2026	**2025**
Cash paid for amounts included in the measurement of lease liabilities		
Operating cash flows from operating leases	$ 767	$ 955
Financing cash flows from finance leases	$ 14	$ —

	Nine-Month Periods Ended September 30,	
	2026	2025
Cash paid for amounts included in the measurement of lease liabilities		
Operating cash flows from operating leases	$ 3,562	$ 3,412
Financing cash flows from finance leases	$ 43	$ —

Future minimum lease payments under non-cancellable leases as of September 30, 2026 are as follows (in thousands):

	Operating Leases	Finance Leases
2026	$ 914	$ 14
2027	3,823	57
2028	3,475	57
2029	2,393	14
2030	1,850	—
Thereafter	4,201	—
Total undiscounted lease payments	$ 16,656	$ 142
Less: Imputed interest	(4,820)	(27)
Total lease liabilities	$ 11,836	$ 115

12. Debt Financing

Notes Purchase Agreement

On August 2, 2022, the Company and the Subsidiary Guarantors entered into the Financing Agreement with Perceptive, which was converted on December 19, 2022 into a Notes Purchase Agreement on substantially the same economic terms and conditions as the Financing Agreement, subject to certain customary note constitution provisions. The Notes Purchase Agreement provided for an initial issuance of $75.0 million of senior secured notes.

During the second quarter of 2026, the Notes Purchase Agreement was amended to extend its maturity date to July 1, 2027 and to require the repayment of $25.0 million of the outstanding principal on or before June 30, 2026. Outstanding borrowings bore interest at a variable annual rate equal to 10.0% plus one-month secured overnight financing rate, subject to a 1.0% floor.

On June 30, 2026, the Company voluntarily repaid all outstanding principal and accrued interest under the Notes Purchase Agreement, resulting in the full repayment and termination of such agreement. No early redemption fee was incurred. Upon termination, all collateral securing the outstanding debt, including the Company's manufacturing facilities in London, United Kingdom and Shannon, Ireland, certain cash balances, the bank accounts of the subsidiary guarantors, and the equity interests of the Subsidiary Guarantors, together with the related security interests and covenants, were released.

During the three months ended September 30, 2026 and 2025, the Company recognized interest expense of nil and $2.7 million, respectively. During the nine months ended September 30, 2026 and 2025, interest expense was $5.2 million and $8.2 million, respectively.

Debt issuance costs, including lender fees and legal costs, were recorded as a discount to the Notes Purchase Agreement and amortized to interest expense over the expected term of the facility using the effective interest method. At June 30, 2026, the remaining unamortized discount of $0.3 million was written off to interest expense in connection with the repayment and termination of the Notes Purchase Agreement.

In connection with entering into the Financing Agreement, the Company granted warrants to Perceptive to purchase up to (i) 400,000 ordinary shares of the Company at an exercise price of $15.00 per share and (ii) 300,000 ordinary

shares of the Company at an exercise price of $20.00 per share. Pursuant to an amendment entered into on March 25, 2026, the warrants were amended to change the exercise price to $8.00 per share. The warrants are exercisable immediately and expire on August 2, 2027.

Royalty Note Purchase Agreement

On June 30, 2026, MeiraGTx, LLC, as issuer, and MeiraGTx Holdings and certain of its subsidiaries entered into a Royalty Note Purchase Agreement (the "Royalty Note Purchase Agreement") with Maverick SA LLC (an affiliate of funds managed by Oberland Capital) as purchaser agent, and certain purchasers ("Purchasers"), pursuant to which the Purchasers purchased an aggregate of $100.0 million of senior secured royalty notes ("Royalty Notes").

Each Purchaser agreed to purchase additional Royalty Notes, subject to the satisfaction of certain conditions and prior to the applicable funding deadline for each purchase tranche. The Purchasers' funding commitments remain available until the earliest of the applicable funding deadline for each purchase tranche or the occurrence of certain specified termination events, including a change of control, repayment or maturity of the outstanding balance, termination of the commitments in accordance with the Royalty Note Purchase Agreement, or the end of the revenue payment period. The additional purchase commitments are as follows:

- a second purchase of an aggregate of $25.0 million of Royalty Notes, which occured in one purchase on July 17, 2026 (the "Second Purchase");
- an additional Purchase of an aggregate of $50.0 million of Royalty Notes in one purchase at MeiraGTx, LLC's option upon the occurrence of positive data readouts from the Phase 2 AQUAx2 study for AAV-hAQP1 for the treatment of radiation-induced xerostomia (the "Third Purchase");
- an additional Purchase of an aggregate of $50.0 million of Royalty Notes in one purchase at MeiraGTx, LLC's option upon receipt of marketing approval from either the U.S. Food and Drug Administration ("FDA") or the European Medicines Agency for bota-vec for the treatment of RPGR gene-associated X-linked retinitis pigmentosa;
- an additional Purchase of an aggregate of $50.0 million of Royalty Notes in one purchase at MeiraGTx, LLC's option upon receipt of marketing approval from the FDA for AAV-hAQP1 for the treatment of radiation-induced xerostomia; and
- an additional Purchase of an aggregate of up to $100.0 million of Royalty Notes prior to December 31, 2028, at the Company's option and subject to the approval of each Purchaser agreeing to participate therein, in its sole discretion.

The Purchasers will be entitled to receive capped payments (the "Revenue Payments") equal to 1.95% of the global net sales ("Net Sales") of the Company's gene therapy product candidates AAV-AIPL1, AAV-hAQP1 and bota-vec (the "Included Products"), which may increase pro rata upon the making of any Purchase subsequent to the aggregate purchases of $125 million in Royalty Notes, and may decrease pro rata upon any voluntary partial repurchase at any time of the Royalty Notes, in each case subject to the applicable cap. If the aggregate amount of Revenue Payments, any milestone payment the Company makes under the Royalty Note Purchase Agreement and voluntary repurchase amounts made by the Company to the Purchasers pursuant to the Royalty Note Purchase Agreement as of December 31, 2031 (the "Test Date") equals or exceeds the amount of the aggregate purchase price for the Royalty Notes paid by the Purchasers (the "Total Funded Amount") to the Company pursuant to the Royalty Note Purchase Agreement (the "Test Date Condition"), the then-applicable percentage of Net Sales payable as Revenue Payments will automatically decrease by a percentage specified in the Royalty Note Purchase Agreement for all subsequent years, subject to the applicable cap. If the Test Date Condition is not satisfied by the Test Date of December 31, 2031, the then-applicable percentage of Net Sales payable as Revenue Payments may increase for all subsequent years, subject to the applicable cap, to a rate that would have provided the Purchasers with 100% of the Total Funded Amount as of the Test Date had such rate applied from the date of the First Purchase through and including the Test Date. The capped Revenue Payments will become payable to the Purchasers on a quarterly basis after marketing approval is received for each Included Product.

The Purchasers have an option to terminate the Royalty Note Purchase Agreement and to require the Company to repurchase the Royalty Notes in full for an amount equal to the Total Funded amount plus an agreed capped multiple upon certain enumerated events of default.

Non-refundable milestone payments, all subject to the applicable cap, are due after the first marketing approval by the FDA, EMA or UK Medicines and Healthcare Products Agency of any product, and increase by an additional amount if the Third Purchase is funded. The repayment amount is due on the maturity date of June 30, 2036 or earlier acceleration for an event of default or change of control. Interest shall accrue on all past due payments immediately upon the occurrence and during the continuance of an event of default.

The Company's obligations under the Royalty Note Purchase Agreement are guaranteed by the Company and certain of its subsidiaries and is secured by the Company's and certain of its subsidiaries' cash, equity interests, receivables, property, plant and equipment and specific assets related to the Included Products.

The Royalty Note Purchase Agreement contains features that affect the amount and timing of future cash flows. The Company elected the fair value option for the entire instrument upon initial recognition. Accordingly, the liability is measured at fair value on a recurring basis, with changes in fair value recognized within financial related costs, net in the condensed consolidated statements of operations and comprehensive (loss) income. During the three and nine months ended September 30, 2026, the Company recognized a gain of $3.1 million change in fair value of Royalty Notes, which reduced financial related costs. This non-cash gain is included in non-cash financing related costs in the condensed consolidated statements of cash flows. The fair value of the liability is determined using valuation techniques that incorporate significant unobservable inputs and is classified as a Level 3 fair value measurement.

On June 30, 2026, the Company also entered into a Securities Purchase Agreement ("Securities Purchase Agreement") with the same Purchasers that purchased the Royalty Notes. Under the Securities Purchase Agreement, the Purchasers purchased an aggregate of 950,570 shares on July 17, 2026 at a price per ordinary share of $10.52, which was the volume-weighted average price per share for the 30 trading days prior to signing of the Securities Purchase Agreement, for aggregate proceeds of $10.0 million. The Company also granted the Purchasers (i) the right ("Right") to purchase a number of ordinary shares ("Right Shares") at a price per share as set forth in the Securities Purchase Agreement and (ii) an opportunity to participate in future financings at the same price and on the same terms as those offered to other participants in the offering ("Offering Participation"). The total Right and Offering Participation amount by all Purchasers may not exceed an aggregate of $15.0 million (Note 5). During the three and nine months ended September 30, 2026, the Company recognized a gain of $0.8 million change in fair value of derivative liability in other income (expense) of the condensed consolidated statements of operations and comprehensive (loss) income, which is included in non-cash other income on the condensed consolidated statements of cash flows.

13. Commitments and Contingencies

The Company has filed claims for research and development tax credits in Ireland for the 2023 and 2024 fiscal years. The total potential benefit associated with the filed claims is approximately $7.3 million. These claims are currently under review by the Irish tax authorities. As this represents the Company's first time claiming such credits in Ireland, and the ultimate approval, timing, and amount of any refund remains uncertain, the Company has not recognized a receivable or related income as of September 30, 2026. Any amounts ultimately approved and received will be recognized in the period in which realization becomes probable and reasonably estimable.

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

You should read the following discussion and analysis of financial condition and operating results together with our financial statements and related notes appearing in this Quarterly Report on Form 10-Q ("Form 10-Q") and those included in our Annual Report on Form 10-K for the year ended December 31, 2025 (the "Form 10-K"). Some of the information contained in this discussion and analysis or set forth elsewhere in this Form 10-Q, including information with respect to our plans and strategy for our business and related financing, includes forward-looking statements that involve risks and uncertainties. As a result of many important factors, including those set forth in the "Risk Factors" section of this Form 10-Q, our actual results could differ materially from the results described in, or implied by, the forward-looking statements contained in the following discussion and analysis. For convenience of presentation some of the numbers have been rounded in the text below. Unless the context requires otherwise, references in this Management's Discussion and Analysis of Financial Condition and Results of Operations to the "Company," "we," "us" and "our" refer to MeiraGTx Holdings plc and its subsidiaries.

Overview

We are a vertically integrated, clinical-stage genetic medicines company with a broad pipeline of four late-stage clinical programs. Each of these programs uses local delivery of small doses, resulting in disease-modifying effects in both inherited and more common diseases, in the eye, radiation-induced xerostomia and Parkinson's disease. We use our innovative technology in optimization of capsids, promoters, and novel translational control elements to develop best-in-class, potent, safe viral vectors. Our broad pipeline is supported by end-to-end in-house manufacturing. We have built the most comprehensive manufacturing capabilities in the industry, including two that are licensed for good manufacturing practices ("GMP") viral vector production and a GMP Quality Control facility with clinical and commercial licensure. In addition, we have developed a proprietary manufacturing platform process over 10 years based on more than 20 different viral vectors with leading yield and quality aspects and commercial readiness. Uniquely, we have developed a novel technology for *in vivo* delivery of any biologic therapeutic using oral small molecules. This transformative riboswitch gene regulation technology allows precise, dose-responsive control of gene expression by oral small molecules. We are focusing the riboswitch platform on the regulated *in vivo* delivery of metabolic peptides, including leptin, GLP-1, GIP, glucagon, amylin and PYY, as well as cell therapy, CAR-T for liquid and solid tumors and autoimmune diseases, and additionally, PNS targets addressing long-term intractable pain. We have developed the technology to apply genetic medicine to common diseases, increasing efficacy, addressing novel targets, and expanding access in some of the largest disease areas where the unmet need remains high.

Our discussion of our financial condition and results of operations is based upon our financial statements, which have been prepared in accordance with generally accepted accounting principles in the United States ("GAAP"). Since our formation, we have devoted substantially all of our resources to developing our technology platform, establishing our GMP viral vector manufacturing facilities and our GMP production facility and developing manufacturing processes, advancing the product candidates in our ophthalmology, salivary gland and neurodegenerative disease programs, building our intellectual property portfolio, organizing and staffing our company, developing our business plan, raising capital, and providing general and administrative support for these operations. To date, we have financed our operations primarily with cash on hand and proceeds from the sales of our equity securities, debt financings, strategic collaborations and asset sales, including (i) upfront and milestone payments in connection with the Collaboration, Option and License Agreement with Janssen Pharmaceuticals, Inc. ("Janssen"), dated as of January 30, 2019, for the research, development and commercialization of gene therapies for the treatment of inherited retinal disease, which also provided us with research funding, (ii) the Asset Purchase Agreement, dated as of December 20, 2023, we entered into with Janssen (the "Original Asset Purchase Agreement") pursuant to which we sold to Janssen botaretigene sparoparvovec, or bota-vec, for the treatment of X-linked retinitis pigmentosa ("XLRP") related to mutations in the retinitis pigmentosa GTPase regulator gene (the "RPGR Product"), and other related assets as described in the Original Asset Purchase Agreement, (iii) the Framework Agreements (as defined below), each dated as of March 9, 2025, we and certain of our affiliates entered into with Hologen Limited ("Hologen") and certain of its affiliates as further described below, and (iv) the Strategic Collaboration and License Agreement, dated as of November 7, 2025 (the "Lilly Collaboration Agreement"), we entered into with Eli Lilly and Company ("Lilly") for the research, development and commercialization of genetic medicines in and related to the area of ophthalmology. As of September 30, 2026, we had cash, cash equivalents and

restricted cash of $186.8 million, as well as a total of $23.4 million billed and unbilled receivables from Lilly and Reogen in connection with research and development and transition services and contract manufacturing processes.

We are a clinical stage company and have not generated any product revenues to date. We have ongoing clinical development programs and a broad pipeline of preclinical programs. Since inception, we have incurred significant operating losses. Our net loss for the three-month periods ended September 30, 2026 and September 30, 2025 were $51.3 million and $50.5 million, respectively. Our net income for the nine-month period ended September 30, 2026 and net loss for the nine-month period ended September 30, 2025 were $63.1 million and $129.3 million, respectively. As of September 30, 2026, we had an accumulated deficit of $753.0 million. We do not expect to generate revenue from sales of products unless and until we successfully initiate and complete clinical development and obtain regulatory approval for any product candidates, or satisfy our third party obligations.

Our total operating expenses for the three-month periods ended September 30, 2026 and 2025 were $54.7 million and $46.5 million, respectively. For the nine-month periods ended September 30, 2026 and 2025, our total operating expenses were $172.8 million and $138.5 million, respectively. We expect to continue incurring costs associated with our clinical activities for AAV-hAQP1 for the treatment of radiation-induced xerostomia and bota-vec for the treatment of XLRP associated with mutations in the *RPGR* gene, as well as for AAV-GAD for the treatment of Parkinson's disease. Certain activities related to the AAV-GAD program are performed under our strategic collaboration with Hologen and are expected to generate service revenue that will fund the related development activities. We also expect to continue to incur costs relating to AAV-AIPL1 for the treatment of LCA4. Certain activities performed under the Lilly Collaboration Agreement and related agreements are expected to generate service revenue that will fund our development and manufacturing activities associated with the program. We also incurred expenses during the nine-month period ended September 30, 2026 and expect to continue to incur expenses related to research activities in additional therapeutic areas to expand our pipeline, developing our potentially transformative gene regulation technology, hiring additional personnel as needed in manufacturing, research, clinical operations, quality, commercialization and other functional areas, and associated cash and share-based compensation expense, as well as the further development of internal manufacturing capabilities and capacity and other associated costs including the management of our intellectual property portfolio.

We will require additional capital in the future, which we may raise through equity offerings (including our "at-the-market" equity offering program), debt financings, marketing and distribution arrangements and other collaborations, strategic alliances and licensing arrangements or other sources to enable us to complete the development and potential commercialization of our product candidates. Furthermore, we expect to continue incurring costs associated with being a public company. Adequate additional financing may not be available to us on acceptable terms, or at all. Our failure to raise capital as and when needed would have a negative effect on our financial condition and our ability to pursue our business strategy. In addition, attempting to secure additional financing may divert the time and attention of our management from day-to-day activities and harm our product candidate development efforts. If we are unable to raise capital when needed or on acceptable terms, we would be forced to delay, reduce or eliminate certain of our research and development programs.

Based on our cash, cash equivalents, accounts receivable, accounts receivable – related party, unbilled receivables – related party, unbilled receivables and tax incentive receivable at September 30, 2026, together with the additional $95.0 million upfront payment due from Hologen and associated reimbursements, we estimate that such funds will be sufficient to enable us to fund our operating expenses and capital expenditure requirements into the second half of 2028. This estimate does not include the $135.0 million in potential near-term cash consideration from Lilly upon achievement of certain development and regulatory approval milestones, or any subsequent tranches available under the Royalty Note Purchase Agreement. We have based these estimates on assumptions that may prove to be wrong, and we may use our available capital resources sooner than we currently expect. See "Liquidity and Capital Resources." Because of the numerous risks and uncertainties associated with the development of our product candidates, any future product candidates, our platform and technology and because the extent to which we may enter into collaborations with third parties for development of any of our product candidates is unknown, we are unable to estimate the amounts of increased capital outlays and operating expenses associated with completing the research and development of our product candidates.

Adequate additional funds may not be available to us on acceptable terms, or at all. To the extent that we raise additional capital through the sale of equity or convertible securities, your ownership interest will be diluted, and the terms of these securities may include liquidation or other preferences that adversely affect your rights as a shareholder. Any future debt financing or preferred equity or other financing, if available, may involve agreements that include covenants limiting or restricting our ability to take specific actions, such as incurring additional debt, making capital expenditures or declaring dividends and may require the issuance of warrants, which could potentially dilute your ownership interests.

If we raise additional funds through collaborations, strategic alliances, or licensing arrangements with third parties, we may have to relinquish valuable rights to our technologies, future revenue streams, research programs 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 programs or any future commercialization efforts or grant rights to develop and market product candidates that we would otherwise prefer to develop and market ourselves.

Because of the numerous risks and uncertainties associated with drug development, we are unable to predict the timing or amount of increased expenses or when or if we will be able to achieve or maintain profitability. Even if we are able to generate revenue from product sales, we may not become profitable. If we fail to become profitable or are unable to sustain profitability on a continuing basis, then we may be unable to continue our operations at planned levels and be forced to reduce or terminate our operations.

Asset Purchase and Related Agreements with Janssen Pharmaceuticals, Inc.

On April 15, 2026 (the "Janssen Closing Date"), we and MeiraGTx Ocular UK Limited ("MeiraGTx Ocular") entered into and consummated an Asset Purchase Agreement (the "Asset Purchase Agreement") with Janssen pursuant to which Janssen sold and assigned to us, and we purchased and assumed, that certain License Agreement, dated February 5, 2019, by and between UCLB Business Plc (now UCL Ventures Ltd.) and Janssen (the "UCLB License Agreement"), relating to the research, development, manufacture and exploitation of the RPGR Product and other related assets as described in the Asset Purchase Agreement.

We made an upfront cash purchase price of $25.0 million to Janssen. Additionally, pursuant to and subject to the terms and conditions set forth in the Asset Purchase Agreement, we agreed to pay Janssen a one-time, future contingent consideration of $50.0 million upon both of the following milestones being achieved: (i) our or our affiliates' receipt of regulatory approval for an RPGR Product in the United States and (ii) aggregate net sales by us or our affiliates of all RPGR Products in the United States since the Janssen Closing Date first exceeds $250.0 million. We have also agreed to pay Janssen royalties, based on future net sales globally of the RPGR Product by us or our affiliates, in the mid-teens percentage of annual net sales for the RPGR Product commencing on or after July 1, 2029. Additionally, in the event we or any of our affiliates may receive payments from a third party if we or any of our affiliates grant any license or right to develop or commercialize any RPGR Product to such third party, we will pay a portion of upfront and milestone payments to Janssen, as well as make royalty payments to Janssen for a given RPGR Product based on (A) royalty payments we or our affiliates may receive from such third party (after deduction of any royalty payments due under the UCLB License Agreement) and (B) net sales of a given RPGR Product by such third party.

In connection with the entering into the Asset Purchase Agreement, we and Janssen entered into a Termination Agreement on the Janssen Closing Date (the "Termination Agreement") terminating the Original Asset Purchase Agreement pursuant to which we sold the RPGR Product to Janssen. The Termination Agreement also terminated that certain Supply Agreement, dated as of December 20, 2023 by and between MeiraGTx UK II and Janssen, and certain other documents related to the Original Asset Purchase Agreement.

Johnson & Johnson Innovation – JJDC, Inc. ("JJDC"), the investment arm of Johnson & Johnson and owner of Janssen, owns more than 5% of our outstanding shares. JJDC and Janssen have agreed not to sell or transfer any of our ordinary shares or securities convertible into, exchangeable for, or exercisable for our ordinary shares, for twelve months

after the Janssen Closing Date and following such twelve month period, if Janssen ever intends to sell our shares after the twelve month period, they will provide written notice to us at least five business days prior to taking any action.

Eli Lilly and Company Collaboration Agreement

On November 7, 2025 (the "Lilly Effective Date"), our affiliates MeiraGTx Ocular, MeiraGTx Limited and MeiraGTx UK II Limited ("MeiraGTx UK II"), entered into the Lilly Collaboration Agreement with Lilly for the research, development and commercialization of genetic medicines in and related to the area of ophthalmology. Under the Lilly Collaboration Agreement, MeiraGTx has granted Lilly exclusive, worldwide rights to research, develop and commercialize our product candidate AAV-AIPL1, which treats Leber congenital amaurosis 4, or LCA4, caused by mutations in the AIPL1 gene, as well as two other preclinical product candidates which are intended to treat other inherited retinal dystrophies. Additionally, Lilly has (i) an exclusive license to proprietary intravitreal capsids for use with up to five targets, relating to or useful in the field of ophthalmology, to be selected by Lilly, (ii) an exclusive license to proprietary pan-retinal or rod-specific promoters for use with up to five targets, relating to or useful in the field of ophthalmology, to be selected by Lilly and (iii) a right of first designation with respect to certain target-specific transactions that MeiraGTx Ocular or its affiliates may seek to pursue in the field of ophthalmology. Lilly also has a right of first negotiation for use of our proprietary riboswitch technology in the field of ophthalmological gene editing.

Under the terms of the Lilly Collaboration Agreement, we received an upfront payment of $75.0 million and will be eligible to receive up to over $400.0 million in total milestone payments, including up to $135.0 million in other potential near-term cash consideration upon the achievement of certain development and regulatory approval milestones. Lilly has the right to research, develop and commercialize products under the Lilly Collaboration Agreement, at its own cost. We may also perform research, development and manufacturing services for Lilly under the Lilly Collaboration Agreement and related agreements, for which we are entitled to reimbursement in accordance with the applicable contractual terms. The Lilly Collaboration Agreement also provides for tiered royalties to be paid to MeiraGTx Ocular on future product sales.

Hologen Transactions

On March 9, 2025, we and certain of our affiliates entered into a strategic collaboration with Hologen and certain of its affiliates to advance the development of certain central nervous system gene therapy programs and to support the Company's manufacturing capabilities. Hologen is a leading developer of multi-modal generative AI foundation models of real-world clinical data for clinical medicine and pharmaceutical drug development. As part of the strategic collaboration, Hologen committed to provide an upfront cash payment of $200.0 million and up to an additional $230.0 million to finance the development of our AAV-GAD program in Parkinson's disease to commercialization, as well as other locally-delivered therapies in the central nervous system. As part of the strategic collaboration, we also received an aggregate of 500,000 Class A shares of Hologen at a nominal price. As of September 30, 2026, Hologen has funded $105.0 million of the upfront payment commitment.

The collaboration is governed by two framework agreements. The first, between the Company, MeiraGTx Neuro UK Limited ("MeiraGTx Neuro UK"), Reogen Limited (formerly Hologen Neuro AI Limited) ("Reogen"), and Hologen, relates to the management of the business and affairs of Reogen, including the research, development, manufacture and commercialization of our (i) AAV-GAD investigational gene therapy for the treatment of Parkinson's disease, AAV-BDNF investigational gene therapy for the treatment of genetic obesity disorders and other potential locally delivered genetic medicines to the central nervous system (the "Clinical Programs") and (ii) proprietary device designed to effect the local delivery of a gene therapy product into the central nervous system or any topographic or subcutaneous tissue modification on the face and scalp, of humans or animals (the "Delivery Device"), in each case, in accordance with the terms and conditions of the Reogen Collaboration Agreement as further described below.

The second, between MeiraGTx Manufacturing Limited ("MeiraGTx Manufacturing"), MeiraGTx Limited and Hologen, provides for the management of the business and affairs of MeiraGTx Manufacturing, including Hologen's investment in MeiraGTx Manufacturing and its participation in funding the manufacturing business.

In April 2026, the parties completed the initial closing of the strategic collaboration and entered into amendments to the framework agreements to facilitate the initial funding and investment structures. As part of the initial closing, the Company, MeiraGTx Neuro UK, MeiraGTx Neuro I, LLC ("MeiraGTx Neuro US"), Reogen, Hologen Neuro AI UK Limited ("Hologen Neuro UK") and Hologen entered into the Collaboration and License Agreement, dated as of April 20, 2026 (the "Reogen Collaboration Agreement") pursuant to which MeiraGTx Neuro US granted to Reogen and Hologen Neuro UK, subject to the license granted by Reogen and Hologen Neuro UK back to MeiraGTx Neuro UK, exclusive, worldwide, royalty-free, fully paid-up licenses to certain of our intellectual property rights for the research, development, manufacture and commercialization of the Clinical Programs and the Delivery Device. The parties also completed Hologen's initial investment in MeiraGTx Manufacturing.

Following the initial closing, Hologen will fund an additional $95.0 million to satisfy the remaining portion of the upfront payments provided for under the framework agreements and the Reogen Collaboration Agreement. These funds will be used by Hologen to purchase (i) a portion of the Reogen shares held by MeiraGTx Neuro UK, such that following the purchase, MeiraGTx Neuro UK and Hologen are expected to hold 30% and 70% of the outstanding shares in Reogen, respectively, and (ii) additional shares in MeiraGTx Manufacturing from MeiraGTx Limited, such that following the purchase, Hologen will have increased its minority interest in MeiraGTx Manufacturing. The framework agreement relating to MeiraGTx Manufacturing also provides that, for twelve months after Hologen purchases all of its additional shares using a portion of the proceeds from the remaining amount of the upfront payment to be paid, Hologen will have an option to increase its ownership interest in MeiraGTx Manufacturing to up to 40%, subject to the terms and conditions of the framework agreement. If Hologen does not exercise the option during that twelve month period, then we have an option to purchase all of the shares of MeiraGTx Manufacturing held by Hologen for the same price that Hologen paid for such shares. We may exercise our option beginning on the third anniversary of the date Hologen purchases its additional shares in MeiraGTx Manufacturing and ending three years thereafter.

On September 29, 2026, the Company, MeiraGTx, LLC, MeiraGTx Neuro UK, MeiraGTx Neuro US, Reogen, Hologen Neuro UK and Hologen entered into an Amendment to the Collaboration and License Agreement and Device Assignment and Assumption Agreement (the "Device Assignment Agreement"), pursuant to which the Company assigned certain intellectual property relating to, and contractual rights associated with the development of, the Delivery Device to Reogen, and amended the Reogen Collaboration Agreement to reflect the foregoing.

Recent Development Highlights and Anticipated Milestones

Botaretigene Sparoparvovec (bota-vec) for the Treatment of X-Linked Retinitis Pigmentosa (XLRP):

- The MAA dossier is complete and in publishing and we intend to submit it to the EMA in December 2026, subject to EMA confirmation of the submission date. We anticipate that this will be followed by the submission of the full BLA to the FDA in the first quarter of 2027.
- We hold all rights and interests in bota-vec following our May 2026 acquisition from Janssen.
- XLRP is a rare inherited retinal disease with early onset and progressive degeneration to complete blindness by the third decade of life, with no currently approved treatment options. There are more than 20,000 XLRP-RPGR patients in the U.S. and EU alone.
- The Phase 3 LUMEOS study was a global, randomized study (n=95) in which all patients were treated bilaterally. Data highlight the potential of bota-vec to improve each of the domains of vision, and the safety profile was as expected and manageable, with no new safety signals and an improved inflammatory profile relative to the Phase 1/2 study.
- As the commercial manufacturer of bota-vec, we have successfully completed process performance qualification (PPQ) with Module 3 of the MAA complete. We hold a commercial license for our London, U.K. manufacturing facility and a commercial license for our QC facility in Shannon, Ireland.
- Bota-vec has been granted Fast Track and orphan drug designation from the FDA, and in the EU, has received Priority Medicines (PRIME), advanced therapy medicinal product (ATMP) classification, and orphan drug designation from the local regulatory authorities.

AAV2-hAQP1 for the Treatment of Radiation-Induced Xerostomia:

- The pivotal Phase 2 AQUAx2 study (NCT05926765), a randomized, double-blind, placebo-controlled study at 30 sites in the U.S., Canada, and the U.K., completed enrollment in the second quarter of 2026 and remains on track for a 12-month pivotal data readout in mid-2027 which, if positive, could support a BLA submission by the end of 2027, with potential U.S. approval in 2028.
- The FDA granted Breakthrough Therapy Designation (BTD) to AAV2-hAQP1 in March 2026 for the treatment of grade 2 and grade 3 radiation-induced xerostomia, supported by three-year data from the 24-patient Phase 1 AQUAx study. Results demonstrated sustained, clinically meaningful improvements in both patient-reported outcomes and objective measures of salivary flow, with durable effects maintained from 12 months through 36 months post-treatment, and AAV2-hAQP1 continued to be safe and well-tolerated at each dose tested.
- In October 2026, we presented a poster at the European Society of Gene and Cell Therapy (ESGCT) 2026 Annual Congress (P0096) describing the development of a commercial *in-vitro* biological activity potency assay for AAV-AQP1. The calcein quenching-based, plate reader assay measures water transport in a surrogate cell line, with intra-assay variability below 20% CV, and is being validated to ICH Q2(R2) standards to support Quality Control release and future commercial licensing.

AAV-GAD for the Treatment of Parkinson's Disease:

- The FDA granted Regenerative Medicine Advanced Therapy (RMAT) designation to AAV-GAD for the treatment of Parkinson's disease not adequately controlled with medication in 2025, based on data demonstrating statistically significant efficacy in 2 double-blind sham-surgery controlled studies: a Phase 2 study (n=45) and a Phase 1/2 clinical bridging study (n=14) following the successful Phase 1 dose escalation study (n=14).
- The RMAT application also included analyses using novel AI technology developed by our JV partner, Hologen Limited, which demonstrated potential disease modification resulting from treatment.
- The JV with Hologen, Reogen Limited (Reogen), intends to initiate a Phase 3 study of AAV-GAD in the first quarter of 2027.

AAV-AIPL1 for LCA4:

- We have granted Eli Lilly and Company (Lilly) worldwide exclusive rights to AAV-AIPL1 for the treatment of Leber congenital amaurosis 4 (LCA4). Lilly also received exclusive access rights to certain of our gene therapy technologies for ophthalmology, and certain rights to our riboswitch technology for gene editing in the eye.
- We received an upfront payment of $75 million and are eligible to receive over $400 million in total milestone payments, as well as tiered royalties on licensed products.

Riboswitch Gene Regulation Technology Platform for *in vivo* Delivery:

- Our riboswitch technology is a broadly applicable platform that provides a precise dose of any biologic therapeutic encoded by a transgene in response to a daily oral pill.
- We have demonstrated that the platform is gene agnostic and can be incorporated into any sequence delivered by any vector including lentivirus, AAV, CRISPR or LNPs providing a powerful mechanism for precisely controlling the level and timing of the production of biologic therapeutics in the body.
- We have prioritized our Ribo-Leptin program, followed by a second program focused on neuropathic pain, for planned IND submissions for first-in-human studies using both vector and small molecule.
- In order to expedite the use of our proprietary, optimized small molecule for both of these riboswitch targets, a single ascending dose/multiple ascending dose (SAD/MAD) study of our lead small molecule is expected to initiate in December 2026. Data from this study will inform dose selection for the planned first-in-human combination studies for each of the prioritized riboswitch programs next year.

Corporate and Leadership Updates

- In July 2026, we appointed John Knighton as Executive Vice President of Global Manufacturing and Supply Chain. Mr. Knighton brings more than 30 years of experience from Janssen and GlaxoSmithKline. Most recently, he served as Vice President, Cell & Gene Therapy API Development at Janssen Pharmaceuticals, Inc., where he led a team that manufactured for the approval and commercialization of CARVYKTI® (a personalized CAR T-cell immunotherapy used to treat adult patients with relapsed or refractory multiple myeloma).
- In September 2026, we appointed Alan M. Sebulsky to our Board of Directors. Mr. Sebulsky has more than 40 years of experience in public company investment, financial strategy, and board leadership, most recently serving as Partner and Portfolio Manager at Adage Capital Management for 14 years. He is the Managing Member of Apothecary Capital, serves on the Board of Directors of Parabilis Medicines, Inc., and previously served on the boards and audit committees of Jazz Pharmaceuticals and Arrow International.

Components of Our Results of Operations

Service Revenue

Our service revenue consists of revenue recognized for services performed under collaboration arrangements with third parties. During the current period, service revenue primarily relates to contract manufacturing services provided to Lilly under the collaboration agreement and related agreements.

Service Revenue – Related Party

Our service revenue – related party consists of revenue recognized for services performed under collaboration arrangements with related parties. During the current period, service revenue – related party primarily relates to research and development services provided to Reogen and the recognition of the remaining deferred revenue associated with the Original Asset Purchase Agreement and related agreements with Janssen, as the remaining performance obligations were extinguished upon termination of the agreements. In the prior period, service revenue – related party primarily related to the PPQ services performed in connection with the Original Asset Purchase Agreement and related agreements.

License Revenue – Related Party

Our license revenue – related party consisted of the revenue recognized from the grant of intellectual property licenses under our collaboration arrangements. During the current period, license revenue – related party primarily relates to the licenses granted to Reogen for the Clinical Programs and the Delivery Device.

Operating Expenses

Operating expenses include the following:

Cost of Service Revenue

Our cost of services revenue consists of costs incurred in providing services under collaboration arrangements with third parties. During the current period, cost of service revenue primarily relates to contract manufacturing services performed for Lilly under the collaboration agreement and related agreements.

Cost of Service Revenue – Related Party

Our cost of service revenue – related party consists of costs incurred in providing services under collaboration agreements with related parties. During the current year, cost of service revenue –related party primarily relates to research and development services performed for Reogen. In the prior year cost of service revenue – related party primarily related to the PPQ services performed in connection with the Original Asset Purchase Agreement and related agreements.

General and Administrative Expenses

General and administrative expenses consist primarily of salaries and other related costs, including share-based compensation, for personnel in our executive, finance, legal, business development and administrative functions. General and administrative expenses also include legal fees relating to intellectual property and corporate matters; professional fees for accounting, auditing, tax and consulting services; insurance costs; travel expenses; and office facility-related expenses, which include direct depreciation costs.

We have incurred, and expect to continue to incur, increased expenses associated with being a public company, including costs of accounting, audit, legal, regulatory and tax-related services associated with maintaining compliance with Nasdaq and SEC requirements; director and officer insurance costs; and investor and public relations costs.

Research and Development Expenses

Research and development expenses consist primarily of costs incurred for our research activities, including our discovery efforts, and the development of our product candidates, and include:

- employee-related expenses, including salaries, benefits and travel of our research and development personnel;
- expenses incurred in connection with third-party vendors that conduct clinical and preclinical studies and manufacture the drug product for the clinical trials and preclinical activities;
- costs associated with clinical and preclinical activities including costs related to facilities, supplies, rent, insurance, certain legal fees, share-based compensation, and depreciation; and
- expenses incurred with the development and operation of our manufacturing facilities.

We expense research and development costs as incurred.

Research and development activities are central to our business model. We expect to continue incurring increasing research and development costs associated with our clinical activities for AAV-hAQP1 for the treatment of radiation-induced xerostomia and bota-vec for the treatment of XLRP associated with mutations in the RPGR gene. We also expect to continue to incur expenses related to research activities in additional therapeutic areas to expand our pipeline and develop our potentially transformative gene regulation technology.

We cannot determine with certainty the duration and costs of future clinical trials of our product candidates or any other product candidate we may develop or if, when, or to what extent we will generate revenue from the commercialization and sale of any product candidate for which we obtain marketing approval. We may never succeed in obtaining marketing approval for any product candidate. The duration, costs and timing of clinical trials and development of our existing product candidates or any other product candidate we may develop will depend on a variety of factors, including:

- the scope, rate of progress, expense and results of clinical trials of our existing product candidates, as well as of any future clinical trials of other product candidates and other research and development activities that we may conduct;
- uncertainties in clinical trial design and patient enrollment rates;
- the actual probability of success for our product candidates, including the safety and efficacy, early clinical data, competition, manufacturing capability and commercial viability;
- significant and changing government regulation and regulatory guidance;
- the timing and receipt of any marketing approvals; and

- the expense of filing, prosecuting, defending and enforcing any patent claims and other intellectual property rights.

A change in the outcome of any of these variables with respect to the development of a product candidate could mean a significant change in the costs and timing associated with the development of that product candidate. For example, if the U.S. Food and Drug Administration (the "FDA") or another U.S. or foreign regulatory authority were to require us to conduct clinical trials beyond those that we anticipate will be required for the completion of clinical development of a product candidate, or if we experience significant delays in our clinical trials due to patient enrollment or other reasons, we would be required to expend significant additional financial resources and time on the completion of clinical development.

Other Non-Operating Income (Expense)

Other non-operating income (expense) includes the following:

Foreign Currency (Loss) Gain

Our condensed consolidated financial statements are presented in U.S. dollars, which is our reporting currency. The financial position and results of operations of our subsidiaries are measured using the foreign subsidiaries' local currency as the functional currency, either the pound sterling or the euro. These entities' cash accounts holding U.S. dollars and intercompany payables and receivables are remeasured based upon the exchange rate at the date of remeasurement with the resulting gain or loss included in the condensed consolidated statements of operations and comprehensive (loss) income.

Interest Income

Interest income is comprised of interest earned on our interest-bearing bank accounts.

Financial Related Costs, net

Financial related costs, net consist primarily of financing costs expensed in connection with the Royalty Notes, changes in the fair value of the Royalty Notes, as well as interest expense, and amortization of the debt discount in connection with the Notes Purchase Agreement. The Notes Purchase Agreement and Royalty Notes are further described in Note 12 to our unaudited condensed consolidated financial statements elsewhere in this Form 10-Q.

Other Income (Expense)

Other income (expense) primarily reflects changes in the fair value of the contingent forward instrument, derivative liability, and equity option instrument. The derivative liability relates to the contractual right granted to Oberland Capital Management LLC ("Oberland Capital") under the Securities Purchase Agreement as further described in Note 12 to our unaudited condensed consolidated financial statements included elsewhere in this Form 10-Q. Other expense also includes a non-cash loss resulting from the initial recognition of the derivative liability at fair value upon execution of the agreement.

Loss on Equity Method Investee

Loss on equity method investee represents our share of the operating results of Reogen, which is accounted for under the equity method of accounting. The amount reflects our proportionate share of Reogen's net loss for the reporting period.

Income Tax Expense

Income tax expense represents current and deferred income taxes recognized on our earnings and losses for the period, including the impact of valuation allowances and discrete tax items.

Other Comprehensive (Loss) Gain

Other comprehensive (loss) gain includes the following:

Foreign Currency Translation Gain (Loss)

Revenue and expenses of subsidiaries have been translated into U.S. dollars at average exchange rates prevailing during the period. Assets and liabilities have been translated at the rates of exchange on the condensed consolidated balance sheet date. The resulting translation gain and loss adjustments are recorded directly as a separate component of shareholders' equity and as other comprehensive (loss) gain on the condensed consolidated statements of operations and comprehensive (loss) income.

Net Loss Attributable to Non-Controlling Interest

Net loss attributable to non-controlling interests represents the portion of losses attributable to equity interests in subsidiaries that are not wholly-owned by us. During the current period, non-controlling interests primarily related to Hologen's minority ownership interest in MeiraGTx Manufacturing following the initial closing of the strategic collaboration.

Critical Accounting Policies and Use of Estimates

Management's discussion and analysis of our financial condition and results of operations is based on our condensed consolidated financial statements, which have been prepared in accordance with GAAP. The preparation of these condensed consolidated financial statements requires us to make estimates and judgements that affect the reporting amounts of assets, liabilities, revenues and expenses and the disclosure of contingent assets and liabilities in our condensed consolidated financial statements. On an ongoing basis, we evaluate our estimates and judgements, including those related to measurement of progress towards satisfaction of performance obligations, share-based compensation and accrued expenses. We base our estimates on historical experience, known trends and events and various other factors that we believe to be reasonable under the circumstances, the results of which form the basis for making judgements about the carrying value of assets and liabilities that are not readily apparent from our sources. Actual results may differ from these estimates under different assumptions.

The Company's critical accounting policies, significant judgements and estimates are included in the Company's Form 10-K for the year ended December 31, 2025 and Note 2 to our unaudited condensed consolidated financial statements included elsewhere in this Form 10-Q.

Results of Operations

Comparison of Three Months Ended September 30, 2026 and 2025

	2026	2025	Change
Revenues:			
Service revenue	$ 1,571	$ —	$ 1,571
Service revenue - related party	3,243	410	2,833
Total revenue	4,814	410	4,404
Operating expenses:			
Cost of service revenue	617	—	617
Cost of service revenue - related party	2,806	313	2,493
General and administrative	11,953	13,616	(1,663)
Research and development	39,316	32,532	6,784
Total operating expenses	54,692	46,461	8,231
Loss from operations	(49,878)	(46,051)	(3,827)
Other non-operating income (expense):			
Foreign currency loss	(1)	(1,558)	1,557
Interest income	1,150	161	989
Financial related costs, net	1,635	(3,065)	4,700
Other income	355	—	355
Loss on equity method investee	(3,929)	—	(3,929)
Income tax expense	(613)	—	(613)
Net loss	$ (51,281)	$ (50,513)	$ (768)

Service Revenue

Service revenue was $1.6 million for the three months ended September 30, 2026, compared to nil for the three months ended September 30, 2025. The increase of $1.6 million was due to revenue recognized for the contract manufacturing services provided to Lilly, as well as other support services related to the AAV-AIPL1 program, under the Lilly Collaboration Agreement and related agreements.

Service Revenue – Related Party

Service revenue – related party was $3.2 million for the three months ended September 30, 2026, compared to $0.4 million for the three months ended September 30, 2025. The increase of $2.8 million was primarily attributable to the development and transition services provided to Reogen under the Reogen Collaboration Agreement and related agreements in the current period, compared to PPQ services provided to Janssen under the Original Asset Purchase Agreement and related agreements in 2025.

Cost of Service Revenue

Cost of service revenue was $0.6 million for the three months ended September 30, 2026 compared to nil for the three months ended September 30, 2025. The increase of $0.6 million was attributable to costs incurred during the three months ended September 30, 2026 in connection with contract manufacturing services provided to Lilly, as well as other related costs associated with support services for the AAV-AIPL1 program under the Lilly Collaboration Agreement and related agreements.

Cost of Service Revenue – Related Party

Cost of service revenue – related party was $2.8 million for the three months ended September 30, 2026 compared to $0.3 million for the three months ended September 30, 2025. The increase of $2.5 million was primarily attributable to costs incurred during the current period related to the development and transition services provided to

Reogen under the Reogen Collaboration Agreement and related agreements, compared to costs associated with PPQ services provided to Janssen under the Original Asset Purchase Agreement and related agreements in 2025.

General and Administrative Expenses

General and administrative expenses were $12.0 million for the three months ended September 30, 2026, compared to $13.6 million for the three months ended September 30, 2025. The decrease of $1.6 million was primarily due to $1.2 million decrease in legal fees, $0.9 million decrease in facilities costs, $0.3 million decrease in consulting fees, and a $0.8 million decrease resulting from the allocation of certain general and administrative expenses to cost of service revenue and cost of service revenue – related party. These decreases were partially offset by $1.6 million increase in personnel costs.

Research and Development Expenses

Research and development expenses for the three months ended September 30, 2026 and 2025 were as follows (in thousands):

	Three-Month Periods Ended September 30,		
	2026	2025	Change
Clinical Programs			
Botaretigene sparoparvovec	$ 2,321	$ —	$ 2,321
AAV-hAQP1	6,163	1,997	4,166
AAV-GAD	4,556	1,200	3,356
Other ocular diseases	—	2,915	(2,915)
Manufacturing	16,664	15,987	677
Preclinical Programs			
Gene regulation	2,732	2,683	49
Neurodegenerative diseases	323	271	52
Ocular diseases	23	688	(665)
Other research and development expenses	6,534	6,791	(257)
Total research and development expenses	$ 39,316	$ 32,532	$ 6,784

Clinical program expenses represent the direct costs for each clinical trial plus the cost of the clinical trial material charged from the manufacturing costs.

Manufacturing expenses represent the costs to manufacture clinical trial material, including payroll, facilities, manufacturing supplies, raw materials, quality control and quality assurance. Upon completion of the manufacture of a batch of clinical trial material, the standard cost of manufacturing the batch of clinical trial material is charged to the clinical programs.

Preclinical program expenses represent the direct costs for each group of preclinical programs.

Other research and development expenses represent costs that are not allocated to a specific clinical or preclinical program, such as payroll and payroll related costs, share-based compensation, travel, rent and facilities costs, depreciation and other non-program specific expenses.

Research and development expenses were $39.3 million for the three months ended September 30, 2026, compared to $32.5 million for the three months ended September 30, 2025. The increase of $6.8 million was primarily attributable to increased clinical trial activities and manufacturing of clinical trial batch material related to our AAV-hAQP1 program, higher expenses associated with our bota-vec program following its reacquisition from Janssen in the

second quarter of 2026, and increased costs associated with our AAV-GAD program, primarily due to the allocation of manufacturing batch costs that were not recognized in cost of service revenue – related party. Manufacturing costs also increased primarily due to scheduled maintenance activities during the quarter, although this increase was fully offset by lower expenses associated with other preclinical ocular programs. In addition, expenses associated with other clinical ocular programs decreased.

Foreign Currency (Loss) Gain

Foreign currency loss was de minimis for the three months ended September 30, 2026 compared to a loss of $1.6 million for the three months ended September 30, 2025. The $1.6 million decrease in foreign currency loss was primarily due to the fluctuations in exchange rates, including the weakening of the U.S. dollar against the pound sterling and the strengthening against the euro which impacted the remeasurement of our intercompany payables and receivables.

Interest Income

Interest income was $1.2 million for the three months ended September 30, 2026 compared to $0.2 million for the three months ended September 30, 2025. The increase of $1.0 million was due to higher cash balances in interest bearing accounts during 2026 offset by lower interest rates.

Financial Related Costs, net

Financial related costs, net reflected a gain of $1.6 million for the three months ended September 30, 2026 compared to an expense of $3.1 million for the three months ended September 30, 2025. The favorable change of $4.7 million was primarily due to a gain from the change in fair value of the Royalty Notes and lower interest expense following the termination of the Notes Purchase Agreement in the second quarter of 2026 as further described in Note 12 to our unaudited condensed consolidated financial statements included elsewhere in this Form 10-Q, partially offset by the transaction cost expensed in connection with the issuance of the Royalty Notes and subsequent change in fair value.

Other Income

Other income was $0.4 million for the three months ended September 30, 2026 compared to nil for the three months ended September 30, 2025. The increase was primarily attributable to a $0.8 million gain from the change in fair value of the derivative liability associated with the right granted under the Securities Purchase Agreement with Oberland Capital and a $0.4 million gain from the change in fair value of the equity option instrument, partially offset by a $0.8 million loss from the change in fair value of the contingent forward instrument.

Loss on Equity Method Investee

Loss on equity method investee was $3.9 million for the three months ended September 30, 2026, compared to nil for the three months ended September 30, 2025. The loss primarily reflects the Company’s proportionate share of Reogen’s expenses associated with the ongoing research and development activities relating to the Reogen Collaboration Agreement.

Income Tax Expense

Income tax expense was $0.6 million for the three months ended September 30, 2026 compared to nil for the three months ended September 30, 2025. The increase of $0.6 million was primarily driven by changes in assumptions used to estimate the annual effective tax rate based on the Company’s forecasted results for the full year.

Comparison of Nine Months Ended September 30, 2026 and 2025

	2026	2025	Change
Revenues:			
Service revenue	$ 13,456	$ —	$ 13,456
Service revenue - related party	108,442	6,027	102,415
License revenue - related party	204,643	—	204,643
Total revenue	326,541	6,027	320,514
Operating expenses:			
Cost of service revenue	2,018	—	2,018
Cost of service revenue - related party	8,767	4,367	4,400
General and administrative	32,894	35,293	(2,399)
Research and development	129,132	98,807	30,325
Total operating expenses	172,811	138,467	34,344
Income (loss) from operations	153,730	(132,440)	286,170
Other non-operating income (expense):			
Foreign currency (loss) gain	(4,304)	10,753	(15,057)
Interest income	2,045	1,540	505
Financial related costs, net	(4,722)	(9,142)	4,420
Other expense	(4,868)	—	(4,868)
Loss on equity method investee	(75,831)	—	(75,831)
Income tax expense	(2,975)	—	(2,975)
Net income (loss)	$ 63,075	$ (129,289)	$ 192,364

Service Revenue

Service revenue was $13.5 million for the nine months ended September 30, 2026, compared to nil for the nine months ended September 30, 2025. The increase of $13.5 million was due to revenue recognized for the contract manufacturing services provided to Lilly, as well as other support services related to the AAV-AIPL1 program, under the Lilly Collaboration Agreement and related agreements.

Service Revenue – Related Party

Service revenue – related party was $108.4 million for the nine months ended September 30, 2026, compared to $6.0 million for the nine months ended September 30, 2025. The increase of $102.4 million was due to the recognition of related party deferred revenue following the termination of the Original Asset Purchase Agreement and Supply Agreement with Janssen and the development and transition services provided to Reogen under the Reogen Collaboration Agreement and related agreements which is partially offset by the decreased activity of PPQ services under the Original Asset Purchase Agreement and related agreements as the agreement was terminated in the second quarter of 2026 and the work was substantially completed in the first half of 2025.

License Revenue – Related Party

License revenue – related party was $204.6 million for the nine months ended September 30, 2026, compared to nil for the nine months ended September 30, 2025. The increase of $204.6 million was due to revenue recognized for the licenses granted to Reogen for the Clinical Programs and the Delivery Device.

Cost of Service Revenue

Cost of service revenue was $2.0 million for the nine months ended September 30, 2026 compared to nil for the nine months ended September 30, 2025. The increase of $2.0 million was due to costs related to contract manufacturing services provided to Lilly, as well as other related costs associated with support services for the AAV-AIPL1 program under the Lilly Collaboration Agreement and related agreements.

Cost of Service Revenue – Related Party

Cost of service revenue – related party was $8.8 million for the nine months ended September 30, 2026 compared to $4.4 million for the nine months ended September 30, 2025. The increase of $4.4 million was due to the development and transition services provided to Reogen under the Reogen Collaboration Agreement and related agreements, partially offset by the decreased activity of PPQ services provided to Janssen under the Original Asset Purchase Agreement and related agreements as the agreement was terminated during the second quarter of 2026 and the work was substantially completed in the first half of 2025.

General and Administrative Expenses

General and administrative expenses were $32.9 million for the nine months ended September 30, 2026, compared to $35.3 million for the nine months ended September 30, 2025. The decrease of $2.4 million was primarily due to a $2.0 million decrease in legal fees, $0.8 million decrease in facilities costs and a $0.8 million decrease resulting from the allocation of certain general and administrative expenses to cost of service revenue and cost of service revenue – related party. In addition, the prior year included a $1.3 million release of asset retirement obligation provisions related to U.S. office and laboratory leases, which did not recur in the current period.

Research and Development Expenses

Research and development expenses for the nine months ended September 30, 2026 and 2025 were as follows (in thousands):

	Nine-Month Periods Ended September 30,		
	2026	2025	Change
Clinical Programs			
Botaretigene sparoparvovec	$ 33,655	$ —	$ 33,655
AAV-hAQP1	15,883	9,603	6,280
AAV-GAD	8,573	11,630	(3,057)
Other ocular diseases	108	4,380	(4,272)
Manufacturing	43,935	45,627	(1,692)
Preclinical Programs			
Gene regulation	5,814	8,210	(2,396)
Neurodegenerative diseases	1,138	995	143
Ocular diseases	1,029	2,475	(1,446)
Other research and development expenses	18,997	15,887	3,110
Total research and development expenses	$ 129,132	$ 98,807	$ 30,325

Clinical program expenses represent the direct costs for each clinical trial plus the cost of the clinical trial material charged from the manufacturing costs.

Manufacturing expenses represent the costs to manufacture clinical trial material, including payroll, facilities, manufacturing supplies, raw materials, quality control and quality assurance. Upon completion of the manufacture of a batch of clinical trial material, the standard cost of manufacturing the batch of clinical trial material is charged to the clinical programs.

Preclinical program expenses represent the direct costs for each group of preclinical programs.

Other research and development expenses represent costs that are not allocated to a specific clinical or preclinical program, such as payroll and payroll related costs, share-based compensation, travel, rent and facilities costs, depreciation and other non-program specific expenses.

Research and development expenses were $129.1 million for the nine months ended September 30, 2026, compared to $98.8 million for the nine months ended September 30, 2025. The increase of $30.3 million was primarily attributable to the reacquisition of bota-vec under the Asset Purchase Agreement and increased costs associated with the

AAV-hAQP1 clinical program, including the manufacturing of clinical trial batch material. These increases were partially offset by lower manufacturing costs, primarily due to increased allocations of manufacturing batch costs to clinical programs and cost of service revenue. In addition, other research and development expenses increased due to higher employee and employee-related costs, facilities costs and other general research and development expenses. These increases were partially offset by lower expenses associated with the AAV-GAD program and other ocular programs, reflecting the allocation of clinical batch manufacturing costs to these programs in 2025, whereas certain costs associated with the AAV-GAD program are now recognized in cost of service revenue rather than research and development expenses. Expenses associated with other ocular programs also decreased. Preclinical program expenses decreased primarily due to the completion of certain gene regulation studies in 2025.

Foreign Currency (Loss) Gain

Foreign currency loss was $4.3 million for the nine months ended September 30, 2026 compared to a gain of $10.8 million for the nine months ended September 30, 2025. The $15.1 million decrease in foreign currency loss was primarily attributable to fluctuations in exchange rates, including the weakening of the U.S. dollar against the pound sterling and strengthening against the euro as it relates to the valuation of our intercompany payables and receivables.

Interest Income

Interest income was $2.0 million for the nine months ended September 30, 2026 compared to $1.5 million for the nine months ended September 30, 2025. The increase of $0.5 million was due to higher cash balances held in interest bearing accounts during the second and third quarters of 2026.

Financial Related Costs, net

Financial related costs, net was $4.7 million for the nine months ended September 30, 2026 compared to $9.1 million for the nine months ended September 30, 2025. The decrease of $4.4 million was primarily due to lower interest expense following the termination of the Notes Purchase Agreement in the second quarter of 2026 as further described in Note 12 to our unaudited condensed consolidated financial statements included elsewhere in this Form 10-Q, and a gain from the change in fair value of the Royalty Notes, partially offset by the transaction cost expensed in connection with the issuance of the Royalty Notes and subsequent change in fair value.

Other Expense

Other expense was $4.9 million for the nine months ended September 30, 2026 compared to nil for the nine months ended September 30, 2025. The increase was primarily attributable to a $5.3 million non-cash loss recognized upon the initial recognition of the derivative liability associated with the right granted under the Securities Purchase Agreement with Oberland Capital and a $0.8 million loss from the change in fair value of the contingent forward instrument. These losses were partially offset by a $0.8 million gain from the change in fair value of the derivative liability and a $0.4 million gain from the change in fair value of the equity option instrument.

Loss on Equity Method Investee

Loss on equity method investee was $75.8 million for the nine months ended September 30, 2026, compared to nil for the nine months ended September 30, 2025. The loss primarily reflects the Company's proportionate share of Reogen's expense associated with the acquired in-process research and development assets relating to the Clinical Programs and the Delivery Device, as well as Reogen's ongoing research and development activities under the Reogen Collaboration Agreement.

Income Tax Expense

Income tax expense was $3.0 million for the nine months ended September 30, 2026 compared to nil for the nine months ended September 30, 2025. The increase of $3.0 million was primarily attributable to higher forecasted taxable income, driven by strategic collaboration transactions and changes in assumptions used to determine the estimated annual effective tax rate applied to year-to-date results.

Liquidity and Capital Resources

Since our inception, we have incurred significant operating losses. For the nine months ended September 30, 2026, we had $45.3 million used by cash flows from operations. There are no assurances that we will generate positive cash flows in the future. Additionally, there are no assurances that we will be successful in obtaining an adequate level of financing for the development and commercialization of our product candidates. We expect to incur significant expenses and operating losses for the foreseeable future as we advance the preclinical and clinical development of our product candidates. We expect that our research and development and general and administrative costs will increase in connection with conducting preclinical studies and clinical trials for our product candidates, building out internal capacity to have products manufactured to support preclinical studies and clinical trials as well as to manufacture commercial products, expanding our intellectual property portfolio, and providing general and administrative support for our operations. As a result of these incurred and expected expenses we will need additional capital to fund our operations, which we may obtain from additional equity or debt financings, collaborations, licensing arrangements, or other sources.

We do not currently have any approved products and have never generated any revenue from product sales. We have historically financed our operations primarily through cash on hand and proceeds from the sale of our equity securities, issuance of debt and upfront and milestone payments from our collaboration and business development activities and asset sales.

Based on our current cash, cash equivalents, accounts receivable, accounts receivable – related party, unbilled receivables – related party, unbilled receivables and tax incentive receivable at September 30, 2026, together with the additional $95.0 million upfront payment due from Hologen and associated reimbursements, we estimate that such funds will be sufficient to enable us to fund our operating expenses and capital expenditure requirements into the second half of 2028. This estimate does not include the $135.0 million in potential near-term cash consideration from Lilly upon achievement of certain development and regulatory approval milestones, or any subsequent tranches available under the Royalty Note Purchase Agreement. We have based these estimates on assumptions that may prove to be wrong, and we could utilize our available capital resources sooner than we expect.

Cash Flows

As of September 30, 2026 we had $186.75 million of cash, cash equivalents and restricted cash.

The following table summarizes our sources and uses of cash, cash equivalents and restricted cash for the period presented:

	For the Nine-Months Ended September 30,	
	2026	2025
	(in thousands)	
Net cash used in operating activities	$ (45,276)	$ (92,990)
Net cash used in investing activities	(27,626)	(3,018)
Net cash provided by financing activities	191,555	6,944
Net increase (decrease) in cash, cash equivalents and restricted cash	$ 118,653	$ (89,064)

Operating Activities

During the nine months ended September 30, 2026, net cash used in operating activities was $45.3 million. This primarily reflects a net income of $63.1 million, which was driven by revenue recognized for the licenses granted for the Clinical Programs and the Delivery Device to Hologen, partially offset by a net total of $32.5 million non-cash adjustments. These non-cash adjustments included $105.0 million of non-cash consideration received from related party – revenue arrangement, $75.8 million of undistributed losses of equity method investee, $27.7 million of acquired in-

process research and development, $15.1 million of share-based compensation, $8.7 million of depreciation and amortization, $4.9 million of gain in non-cash other income, $3.1 million of loss in non-cash financing related costs, $4.3 million of foreign exchange losses, $1.9 million of change in right-of-use assets, $1.5 million of loss on disposal of equipment, furniture and fixtures, and $0.7 million of amortization of debt discount. In addition, changes in working capital affected operating cash flows, with operating assets decreasing by $35.9 million and operating liabilities decreasing by $105.0 million.

During the nine months ended September 30, 2025, our cash used in operating activities of $93.0 million was primarily due to a net loss of $129.3 million as we incurred expenses associated with research activities on our clinical programs, manufacturing of our clinical trial materials, preclinical research programs and general and administrative expenses. The net loss included non-cash income and expense of $12.8 million, which consisted primarily of $16.5 million of share-based compensation, $9.4 million of depreciation and amortization, $0.8 million of amortization of debt discount, $10.8 million of a foreign exchange gain, $2.2 million of change in right-of-use assets and $1.5 million of gain on termination of lease liabilities. Additionally, operating assets, consisting of accounts receivable – related party, contract assets – related party, inventory, prepaid expenses, tax incentive receivable, other assets and other current assets, increased by $1.2 million and operating liabilities, consisting of accounts payable, accrued expenses, other current liabilities and deferred revenue – related party, increased by $22.3 million.

Investing Activities

Net cash used in investing activities for the nine months ended September 30, 2026 was $27.6 million. This primarily consisted of $24.2 million used for the acquisition of in-process research and development assets and $4.1 million of purchases of property and equipment for our manufacturing, laboratory and process development facilities, partially offset by $0.7 million of proceeds from the sale of a non-controlling interest in MeiraGTx Manufacturing to Hologen.

Net cash used in investing activities for the nine months ended September 30, 2025 of $3.0 million consisted of purchases of property and equipment for our manufacturing, laboratory and process development facilities.

Financing Activities

Net cash provided by financing activities was $191.6 million for the nine months ended September 30, 2026, consisting of $170.8 million net proceeds from the issuance of our ordinary shares, and $125.0 million proceeds from the Royalty Notes and $1.4 million exercise of stock options, partially offset by payment of the $75.0 million outstanding principal under the Notes Purchase Agreement, $18.2 million to repurchase 2.3 million of our ordinary shares from Perceptive Life Sciences Master Fund, Ltd. pursuant to a share purchase agreement, $6.5 million of costs associated with the issuance of ordinary shares and $6.0 million paid to satisfy tax withholding obligations upon the vesting of restricted share unit awards.

Net cash provided by financing activities was $6.9 million for the nine months ended September 30, 2025, which consisted of $9.9 million net proceeds from an “at-the-market” offering of our ordinary shares, offset by the payment of $3.0 million to cover tax withholding obligations upon the vesting of restricted share unit awards.

Off-Balance Sheet Arrangements

We have not entered into any off-balance sheet arrangements under applicable SEC rules and do not have any holdings in variable interest entities.

Item 3. Quantitative and Qualitative Disclosures About Market Risk.

The following section updates “Item 7A. Quantitative and Qualitative Disclosures of Market Risk” in the Annual Report on Form 10-K for the fiscal year ended December 31, 2025 and should be read in conjunction with that report as well as our condensed consolidated financial statements included in “Part 1, Item 1. Financial Statements” of this Quarterly Report on Form 10-Q.

Foreign Currency Exchange Risk

We currently operate in the United States, the United Kingdom and the European Union. Our activities in these jurisdictions expose us to currency exchange rate fluctuations, primarily between the U.S. Dollar and the British pound sterling and euro. When the U.S. Dollar strengthens against these currencies, the U.S. Dollar value of non-U.S. Dollar based losses increases. To the extent that our international activities recorded in local currencies increase in the future, our exposure to fluctuations in currency exchange rates will correspondingly increase. As of September 30, 2026, we did not hold any foreign currency forward contracts. With respect to our foreign currency exposures as of September 30, 2026, we estimate a 10% unfavorable movement in foreign currency exchange rates would have the effect of creating an additional foreign currency loss of approximately $14.3 million within other non-operating income (expense) for the nine months ended September 30, 2026.

Interest Rate Risk

Prior to the repayment and termination of the Notes Purchase Agreement on June 30, 2026, we were exposed to market risk as a result of changes in interest rates applicable to borrowings under the Notes Purchase Agreement. As a result, we had no fluctuating rate debt outstanding as of September 30, 2026 and were not exposed to interest rate risk related to borrowings at quarter end.

Item 4. Controls and Procedures.

Limitations on Effectiveness of Controls and Procedures

In designing and evaluating our disclosure controls and procedures, management recognizes that any controls and procedures, no matter how well designed and operated, can provide only reasonable assurance of achieving the desired control objectives. In addition, the design of disclosure controls and procedures must reflect the fact that there are resource constraints and that management is required to apply judgment in evaluating the benefits of possible controls and procedures relative to their costs.

Evaluation of Disclosure Controls and Procedures

Our management, with the participation of our Chief Executive Officer (principal executive officer) and our Chief Financial Officer (principal financial officer), evaluated, as of the end of the period covered by this Form 10-Q, 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”)). Based on that evaluation, our Chief Executive Officer (principal executive officer) and Chief Financial Officer (principal financial officer) concluded that our disclosure controls and procedures were effective at the reasonable assurance level at the end of the period covered by this Form 10-Q.

Changes in Internal Control Over Financial Reporting

There were no changes in our internal control over financial reporting (as defined in Rules 13a-15(f) and 15d-15(f) under the Exchange Act) during the quarter ended September 30, 2026 that have materially affected, or are reasonably likely to materially affect, our internal control over financial reporting.

PART II—OTHER INFORMATION

Item 1. Legal Proceedings.

We are not subject to any material legal proceedings.

ITEM 1A. RISK FACTORS

Investing in our ordinary shares involves a high degree of risk. You should consider carefully the risks described below, together with the other information included or incorporated by reference in this Form 10-Q. If any of the following risks occur, our business, financial condition, results of operations and future growth prospects could be materially and adversely affected. In these circumstances, the market price of our ordinary shares could decline. Other events that we do not currently anticipate or that we currently deem immaterial may also affect our business, prospects, financial condition and results of operations.

Risks Related to Our Financial Position and Need for Additional Capital

We have incurred significant losses since inception and anticipate that we will incur continued losses for the foreseeable future, and may never achieve or maintain profitability.

We are a clinical stage company with limited operating history and have not generated any product revenue to date. We were formed and began operations in 2015 and have incurred significant net losses since inception. Although we reported net income of $63.1 million for the nine month period ended September 30, 2026, such profitability was primarily attributable to non-recurring transactions in the second quarter of 2026. We incurred a net loss of $51.3 million for the three-month period ended September 30, 2026. There is no assurance that profitable operations could be sustained on a continuing basis in the foreseeable future. As of September 30, 2026, we had an accumulated deficit of approximately $753.0 million. Since our inception, we have devoted substantially all of our resources to developing our technology platform, establishing our GMP viral vector manufacturing facilities and GMP DNA production facility, developing manufacturing processes, advancing the product candidates in our ophthalmology, salivary gland and neurodegenerative disease programs, research and development activities, including our riboswitch gene regulation platform technology, building our intellectual property portfolio, organizing and staffing our company, developing our business plans, raising capital, securing debt financing and providing general and administrative support for these operations. We have not yet demonstrated an ability to successfully complete the development of and obtain regulatory approval for any of our product candidates, manufacture product at a commercial scale, or arrange for a third party to do so on our behalf, or conduct sales and marketing activities necessary for successful product commercialization. Given the length of time typically needed to develop a new drug from the time it enters Phase 1 clinical trials to when it is approved for treating patients, if ever, predictions about our future success or viability may not be as accurate as they could be if we had a longer operating history or a history of successfully developing and commercializing genetic medicine products.

We expect to continue to incur significant expenses and additional operating losses in the future as we seek to advance product candidates through preclinical and clinical development, expand our research, development and manufacturing activities, develop new product candidates, build and expand our intellectual product portfolio, complete clinical trials, seek regulatory approval and, if we receive regulatory approval, commercialize our products. Furthermore, the costs of advancing product candidates into each succeeding clinical phase tend to increase substantially over time, including the ongoing Phase 2 AQUAx2 clinical trial of AAV-hAQP1 for the treatment of patients with radiation-induced xerostomia. In addition, we expect to continue incurring increasing research and development costs associated with our clinical activities for AAV-GAD for the treatment of Parkinson’s disease, although certain activities related to the AAV-GAD program are performed under our strategic collaboration with Hologen and are expected to generate service revenue that will fund the related development activities. We also expect to incur increasing research and development costs in connection with research, preclinical and clinical activities for our riboswitch platform. We also expect to incur costs related to the long-term follow up study for patients that enrolled in the Phase 3 LUMEOS clinical trial of bota-vec for the treatment of XLRP, as well as costs related to regulatory activities. The total costs to advance any of our product candidates to marketing approval in even a single jurisdiction would be substantial. Because of the

numerous risks and uncertainties associated with gene therapy product development, we are unable to accurately predict the timing or amount of increased expenses or whether we will be able to begin generating revenue from the commercialization of products or achieve or maintain profitability.

Before we generate any revenue from product sales, each of our programs and product candidates will require additional preclinical and/or clinical development, potential regulatory approval in multiple jurisdictions, manufacturing, building of a commercial organization, substantial investment and significant marketing efforts. Our expenses could increase beyond expectations if we are required by the FDA, UK Medicines and Healthcare products Regulatory Agency (the "MHRA"), European Medicines Agency (the "EMA"), or other regulatory authorities to perform preclinical studies and clinical trials in addition to those that we currently anticipate. These risks are further described under "—Risks Related to Discovery, Development, Clinical Testing, Manufacturing and Regulatory Approval" and "—Risks Related to Commercialization." As a result, we expect to continue to incur net losses for the foreseeable future. These net losses have had, and will continue to have, an adverse effect on our shareholders' equity and working capital.

As we continue to build our business, we expect our financial condition and operating results may fluctuate significantly from quarter to quarter and year to year due to a variety of factors, many of which are beyond our control. Accordingly, you should not rely upon the results of any particular quarterly or annual period as indications of future operating performance. If we are unable to develop and commercialize one or more of our product candidates either alone or with collaborators, or if revenues from any product candidate that receives marketing approval are insufficient, we will not achieve profitability. Even if we do achieve profitability in the future, we may not be able to sustain or increase profitability. If we are unable to achieve and then maintain profitability, the value of our equity securities will be adversely affected.

We will require additional capital to fund our operations, which may not be available on acceptable terms, if at all.

We expect to spend substantial amounts to complete the development of, seek regulatory approvals for and commercialize our product candidates, as well as maintain and/or expand our manufacturing and supply chain capabilities. This will require additional capital, which we may raise through equity offerings, debt financings, additional borrowings under the Royalty Note Purchase Agreement, marketing and distribution arrangements and other collaborations, strategic alliances and licensing arrangements or other sources. Our ability to raise additional capital when needed has been and may in the future be adversely affected by external factors beyond our control, including changes in the political climate, geopolitical actions, changes in market interest rates, potential reforms and changes to government regulations, the effect of healthcare reform legislation, including those that may limit pricing of pharmaceutical products and drugs, market prices and conditions, prospects for favorable or unfavorable clinical trial results, new product initiatives, the manufacturing and distribution of new products, product safety and efficacy issues, new collaborations and strategic alliances and licensing arrangements. Adequate additional financing may not be available to us on acceptable terms, or at all. Our failure to raise capital as and when needed would have a negative effect on our financial condition and our ability to pursue our business strategy. In addition, attempting to secure additional financing has diverted and may in the future divert the time and attention of our management from day-to-day activities and harm our product candidate development efforts. If we are unable to raise capital when needed or on acceptable terms, we would be forced to delay, reduce or eliminate certain of our research and development programs.

Our operations have consumed significant amounts of cash since inception. As of September 30, 2026, our cash, cash equivalents and restricted cash were $186.8 million. Based on our cash, cash equivalents, accounts receivable, accounts receivable – related party, unbilled receivables – related party, unbilled receivables and tax incentive receivable at September 30, 2026, together with the additional $95.0 million upfront payment due from Hologen and associated reimbursements, we estimate that such funds will be sufficient to enable us to fund our operating expenses and capital expenditure requirements into the second half of 2028. This estimate does not include the $135.0 million in potential near-term cash consideration from Lilly upon achievement of certain development and regulatory approval milestones, or any subsequent tranches available under the Royalty Note Purchase Agreement. This estimate is based on assumptions that may prove to be wrong, and we could use our available capital resources sooner than we currently expect. Changing circumstances could cause us to spend more than expected or consume capital significantly faster than we currently anticipate, such as inflation or other factors that may significantly increase our business costs. Because the length of time and activities associated with successful development of our product candidates is uncertain, we are

unable to estimate the actual funds we will require for development and any approved marketing and commercialization activities. Our future funding requirements, both near and long-term, will depend on many factors, including, but not limited to:

- the progress, timing, costs and results of our ongoing clinical development for our X-linked retinitis pigmentosa product candidate, bota-vec, including costs related to the long-term follow up study for patients that enrolled in the Phase 3 LUMEOS clinical trial of bota-vec for the treatment of XLRP, as well as costs related to our regulatory activities and, if bota-vec is approved, costs related to commercialization and any milestone or royalty payments we make to Janssen;

- the progress, timing, costs and results of our clinical development for our radiation-induced xerostomia product candidate, AAV-hAQP1, as well as costs, if approved, related to commercialization of AAV-hAQP1;

- the progress, timing, costs and results of our ongoing clinical development for our AAV-AIPL1 gene therapy product candidate under the Lilly Collaboration Agreement, which costs are expected to be offset by service revenue from Lilly recognized as part of certain development and manufacturing activities we perform under the Lilly Collaboration Agreement and related agreements;

- the progress, timing, costs and results of our clinical development for our product candidate for the treatment of Parkinson's disease, AAV-GAD, although certain of these costs are expected to be offset by service revenue from Hologen recognized as part of certain development activities we perform under the strategic collaboration we entered into with them;

- the development of our transformative riboswitch gene regulation platform technology designed to precisely and specifically control gene therapy expression levels via dose-response to orally delivered small molecules;

- the development of our product candidate for the treatment of ALS, AAV-UPF1, our product candidate for the treatment of xerostomia associated with Sjogren's syndrome, AAV-hAQP1, and our product candidate for the treatment of neovascular age related macular degeneration, or wet AMD;

- the extent to which we receive the milestone payments under the Lilly Collaboration Agreement;

- continuing our current research programs and our preclinical development of product candidates from our current research programs;

- seeking to identify, assess, acquire and/or develop additional research programs and additional product candidates;

- the preclinical testing and clinical trials for any product candidates we identify and develop;

- the outcome, timing and cost of meeting regulatory requirements established by the FDA, MHRA, EMA and other regulatory authorities;

- the cost of expanding and protecting our intellectual property portfolio, including filing, prosecuting, defending and enforcing our patent claims and other intellectual property rights;

- the cost of defending potential intellectual property disputes, including patent infringement actions brought by third parties against us or any of our product candidates;

- the effect of competing technological and market developments;

- the cost of further developing and scaling our manufacturing facilities and processes;
- the cost and timing of completion of commercial-scale manufacturing facilities and activities;
- the cost of making royalty, milestone or other payments under current and any future in-license agreements;
- our ability to establish and maintain strategic collaborations, licensing or other agreements and the financial terms of such agreements;
- the extent to which we in-license or acquire rights to other products, product candidates and technologies;
- the cost of establishing sales, marketing and distribution capabilities for our product candidates in regions where we choose to commercialize our products; and
- the initiation, progress, timing and results of our commercialization of our product candidates, if approved for commercial sale.

Raising additional capital through the sale of equity, convertible debt securities or warrants will dilute your ownership interest, and the terms of these securities may include liquidation or other preferences that adversely affect your rights as a shareholder. Additional debt financing or preferred equity financing, if available, may involve agreements that include covenants further limiting or restricting our ability to take specific actions, such as incurring additional debt, making capital expenditures or declaring dividends. If we raise additional funds through collaborations, strategic alliances or marketing, distribution or licensing arrangements with third parties, we may be required 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.

We may not have sufficient cash flows or cash on hand to satisfy our debt obligations or covenants under our financing arrangements, or we may not be able to effectively manage our business in compliance with such covenants.

On June 30, 2026, we and certain of our subsidiaries entered into a Royalty Note Purchase Agreement (the "Royalty Note Purchase Agreement"), by and among MeiraGTx, LLC, as issuer, MeiraGTx Holdings plc and certain of its subsidiaries, as obligors (collectively, the "Obligors"), the purchasers party thereto (the "Purchasers") and Maverick SA LLC, as purchaser agent, an affiliate of funds managed by Oberland Capital.

Pursuant to the Royalty Note Purchase Agreement, the Purchasers agreed to purchase senior secured royalty notes (the "Royalty Notes") from MeiraGTx, LLC of up to $375 million in a series of purchases of Royalty Note purchases (each, a "Purchase") as follows:

(i) an initial Purchase on June 30, 2026 for an aggregate purchase price of $100 million;

(ii) a second Purchase on July 17, 2026 for an aggregate purchase price of $25 million;

(iii) at MeiraGTx, LLC's option and subject to the satisfaction of certain conditions, an additional Purchase for an aggregate purchase price of $50 million upon the occurrence of positive data readouts from the Phase 2 AQUAx2 study for AAV-hAQP1 for the treatment of radiation-induced xerostomia;

(iv) at MeiraGTx, LLC's option and subject to the satisfaction of certain conditions, an additional Purchase for an aggregate purchase price of $50 million upon bota-vec receipt of marketing approval from either the FDA or the EMA for the treatment of RPGR gene-associated X-linked retinitis pigmentosa;

(v) at MeiraGTx, LLC's option and subject to the satisfaction of certain conditions, an additional Purchase for an aggregate purchase price of $50 million upon AAV-hAQP1's receipt of marketing approval from the FDA for the treatment of radiation-induced xerostomia; and

(vi) at MeiraGTx, LLC's option and subject to the approval of each Purchaser agreeing to participate therein, in its sole discretion, a sixth Purchase for an aggregate purchase price of up to $100 million.

We may enter into a change of control with a third party at any time, and if we consummate a change of control with a third party, we may be required to pay certain specified amounts to Purchasers depending on the timing of such change of control and the identity of such acquiror.

The Purchasers will be entitled to receive capped payments (the "Revenue Payments") equal to 1.95% of the global net sales ("Net Sales") of our gene therapy products AAV-AIPL1, AAV-hAQP1 and bota-vec (the "Included Products") after marketing approval is received for each Included Product, which may increase pro rata upon the making of any Purchase subsequent to the aggregate purchases of $125 million in Royalty Notes, and may decrease pro rata upon any voluntary partial repurchase at any time of the Royalty Notes, in each case subject to the applicable cap. If the aggregate amount of Revenue Payments, any milestone payment we make under the Royalty Note Purchase Agreement and voluntary repurchase amounts we make to the Purchasers pursuant to the Royalty Note Purchase Agreement as of December 31, 2031 (the "Test Date") equals or exceeds the amount of the aggregate purchase price for the Royalty Notes paid by the Purchasers (the "Total Funded Amount") to us pursuant to the Royalty Note Purchase Agreement (the "Test Date Condition"), the then-applicable percentage of Net Sales payable as Revenue Payments will automatically decrease by a percentage specified in the Royalty Note Purchase Agreement for all subsequent years, subject to the applicable cap. If the Test Date Condition is not satisfied by the Test Date of December 31, 2031, the then-applicable percentage of Net Sales payable as Revenue Payments may increase for all subsequent years, subject to the applicable cap, to a rate that would have provided the Purchasers with 100% of the Total Funded Amount as of the Test Date had such rate applied from June 30, 2026 through and including the Test Date.

Non-refundable milestone payments, all subject to the applicable cap, are due after the first marketing approval by the FDA, EMA or MHRA of any product, and increase by an additional amount if the purchase for the positive data readouts from the Phase 2 AQUAx2 study for AAV-hAQP1 is funded. The repayment amount is due on the maturity date of June 30, 2036 or earlier acceleration for an event of default or change of control. Interest shall accrue on all past due payments immediately upon the occurrence and during the continuance of an event of default.

We may elect at any time and at our sole discretion to voluntarily repay in whole or in part in $25 million increments the Total Funded Amount plus agreed capped multiples.

Our obligations under the Royalty Note Purchase Agreement are guaranteed by the Company and certain of its subsidiaries. To secure the Obligors' obligations under the Royalty Note Purchase Agreement, the Obligors have granted a security interest in the Obligors' cash, equity interests, receivables, property, plant and equipment and in specific assets related to the Included Products for the benefit of the Purchasers.

The Royalty Note Purchase Agreement contains various restrictions and covenants on the part of the Obligors, including, among other things, covenants regarding our ability to incur additional indebtedness, grant liens, merge or consolidate, transfer or dispose of assets, make investments, make acquisitions, enter into certain transactions with affiliates and pay dividends or make distributions, in each case, subject to certain exceptions set forth in the Royalty Note Purchase Agreement. The covenants may restrict our current and future operations, particularly our ability to respond to certain changes in our business or industry, or take future actions. Additionally, our ability to comply with these restrictive covenants may be impacted by events beyond our control, such as economic conditions or major central bank policy actions.

The Purchasers have an option to terminate the Royalty Note Purchase Agreement and to require us to repurchase the Royalty Notes in full for an amount equal to the Total Funded Amount plus an agreed capped multiple upon certain enumerated events of default, including, without limitation, our failure to pay certain amounts when due or our breach or failure to satisfy certain restrictive covenants. If the Purchasers were to exercise their option or otherwise declare an event of default under the Royalty Note Purchase Agreement, that could result in foreclosure on all or substantially all of the assets in which we have granted a security interest, which could significantly harm our business, financial condition and results of operations and could potentially result in a substantial or complete loss of your investment in our ordinary shares.

There can be no assurance that our cash and cash equivalents available under the Royalty Note Purchase Agreement and under any future financings, together with any funds generated by our operations, will be sufficient to satisfy our payment obligations. Our inability to generate funds, obtain financing sufficient to satisfy our payment obligations or remain in compliance with the covenants may result in such obligations being accelerated by the Purchasers, which would likely have a material adverse effect on our business, financial condition and results of operations. We may be required to pursue one or more alternatives, such as raising additional capital through equity offerings on terms that may be onerous or highly dilutive, selling assets or restructuring our indebtedness. We may have to relinquish valuable rights to the Included Products, other intellectual property or future revenue streams, or grant licenses on terms that are not favorable to us. Our ability to refinance our indebtedness will depend on the capital and credit markets and our financial condition at such time. If prevailing interest rates or other factors at the time of refinancing result in higher interest rates upon refinancing, then the expense relating to the refinancing would increase. Any of the foregoing risks could materially adversely affect our financial condition, cash flows and results of operations.

Our review of potential strategic transactions may not result in an executed or consummated transaction or other strategic alternative and may not result in anticipated benefits to us or our shareholders, and the process of reviewing strategic transactions or its conclusion could be disruptive and distracting to our business operations and management.

We have, and may continue to, opportunistically identify and evaluate strategic opportunities regarding our assets. Any significant delay in consummating or a failure to consummate or complete a transaction could have a material adverse effect on our future business or operating results. There can be no assurance that we will be successful in our efforts to pursue or advance such options, or identify similar opportunities, or that any potential transaction would be consummated or, if consummated, will provide the anticipated benefits to us or otherwise enhance shareholder value. Any such potential transaction would be dependent upon a number of factors beyond our control, including, without limitation, market conditions, industry trends, the interest of third parties in our assets and whether the terms of any strategic transaction would be acceptable to us. The process of reviewing potential strategic alternatives is time consuming and may be distracting and disruptive to our business operations and long-term planning, which may cause concern to our current or potential customers, employees, investors, strategic partners and other constituencies and may have a material impact on our business and operating results or result in increased volatility in our share price.

We are heavily dependent on the success of our product candidates, which are still in development, and if none of them receive regulatory approval or are successfully commercialized, our business may be harmed.

Our future success and ability to generate product revenue is substantially dependent on our ability to successfully develop, manufacture, obtain regulatory approval for and successfully commercialize our product candidates. We currently have no products that are approved for commercial sale and may never be able to develop marketable products. We have invested and expect to continue to invest a meaningful portion of our efforts and expenditures over the next few years in the development of bota-vec, AAV-hAQP1, AAV-AIPL1, AAV-GAD and our riboswitch gene regulation technology platform, as well as potentially other clinical and pre-clinical programs, which will require additional clinical development, management of clinical and manufacturing activities, regulatory approval in multiple jurisdictions, manufacturing sufficient supply, building of a commercial organization, substantial investment and significant marketing efforts before we can generate any revenues from any commercial sales. While we have entered into the Lilly Collaboration Agreement with respect to AAV-AIPL1 and two other preclinical product candidates which are intended to treat other inherited retinal dystrophies, there can be no assurance that these product candidates will be successfully developed and commercialized by us or Lilly. We cannot be certain that our product candidates will

be successful in clinical trials, receive regulatory approval or be successfully commercialized even if we receive regulatory approval. Even if we receive approval to market our product candidates from the FDA, MHRA or other regulatory bodies, we cannot be certain that our product candidates will be successfully commercialized by us or any of our collaborators, widely accepted in the marketplace or more effective than other commercially available alternatives. Additionally, the research, testing, manufacturing, labeling, approval, sale, marketing and distribution of gene therapy products are and will remain subject to extensive and evolving regulation by the FDA, MHRA and other regulatory authorities. We are not permitted to market our product candidates in the United States until they receive approval of a biologics license application, or BLA, from the FDA, we cannot market them in the UK or European Union, or EU, until we receive approval for a marketing authorization, or MA, from the MHRA or European Commission, respectively, and we cannot market them in other countries until we receive any other required regulatory approval in those countries.

Because some of our product candidates are based on similar technology, if any of our product candidates show unexpected adverse events or a lack of efficacy in the indications we intend to treat, or if we experience other regulatory or developmental issues, our development plans and business could be significantly harmed. Further, competitors may be developing products with similar technology and may experience problems with their products that could identify problems that would potentially harm our business.

We may not be successful in our efforts to identify additional product candidates.

Part of our strategy involves identifying novel product candidates. The process by which we identify product candidates may fail to yield product candidates for clinical development for a number of reasons, including those discussed in these risk factors and also:

- we may not be able to assemble sufficient resources to acquire or discover additional product candidates;
- competitors may develop alternatives that render our potential product candidates obsolete or less attractive;
- potential product candidates we develop may nevertheless be covered by third parties' patents or other exclusive rights;
- potential product candidates may, on further study, be shown to have harmful side effects, toxicities or other characteristics that indicate that they are unlikely to be products that will receive marketing approval and achieve market acceptance;
- potential product candidates may not be effective in treating their targeted diseases;
- the market for a potential product candidate may change so that the continued development of that product candidate is no longer reasonable;
- a potential product candidate may not be capable of being produced in commercial quantities at an acceptable cost, or at all; or
- the regulatory pathway for a potential product candidate may be too complex and difficult to navigate successfully or economically.

In addition, we may choose to focus our efforts and resources on a potential product candidate that ultimately proves to be unsuccessful. As a result, we may fail to capitalize on viable commercial products or profitable market opportunities, be required to forego or delay pursuit of opportunities with other product candidates or other diseases that may later prove to have greater commercial potential, or relinquish valuable rights to such product candidates through collaboration, licensing or other royalty arrangements in cases in which it would have been advantageous for us to retain sole development and commercialization rights. If we are unable to identify additional suitable product candidates for

clinical development, this would adversely impact our business strategy and our financial position and share price and could potentially cause us to cease operations.

Risks Related to Discovery, Development, Clinical Testing, Manufacturing and Regulatory Approval

It is difficult to predict the time and cost of product candidate development on our novel gene therapy platform. A limited number of gene therapies have been approved in the United States or in Europe.

We have concentrated a portion of our research and development efforts on our gene therapy platform, which uses both transduction and gene control technology. Our future success depends on the successful development of these novel therapeutic approaches. To date, a limited number of products that utilize gene transfer have been approved in the United States or Europe.

Our gene therapy platform is based on a suite of viral vectors which we can deploy with gene therapy constructs, which relies on the ability of AAV to efficiently transmit a therapeutic gene to certain kinds of cells. The mechanism of action by which these vectors target particular tissues is still not completely understood. Therefore, it is difficult for us to determine that our vectors will be able to properly deliver gene transfer constructs to enough tissue cells to reach therapeutic levels. We cannot be certain that animal models will exist for some of the diseases we expect to pursue, that our viral vectors will be able to meet safety and efficacy levels needed to be therapeutic in humans or that they will not cause significant adverse events or toxicities. Furthermore, prior work conducted by a third party in non-human primates suggests that intravenous, or IV, delivery of certain AAV vectors at very high doses may result in severe toxicity. The indications that we target do not use IV administration for viral vector delivery and do not use doses as high as those tested in these publications, and to date we have not observed the severe toxicities described in these publications with the naturally occurring AAV vectors that we use. However, we cannot be certain that we will be able to avoid triggering toxicities in our future preclinical studies or clinical trials. Any such results could impact our ability to develop a product candidate. As a result of these factors, it is more difficult for us to predict the time and cost of product candidate development, and we cannot predict whether the application of our gene therapy platform, or any similar or competitive gene therapy platforms, will result in the identification, development, and regulatory approval of any product candidates, or that other gene therapy technologies will not be considered better or more attractive. There can be no assurance that any development problems we experience in the future related to our gene therapy platform or any of our research programs will not cause significant delays or unanticipated costs, or that such development problems can be solved. Any of these factors may prevent us from completing our preclinical studies or clinical trials or commercializing any product candidates we may develop on a timely or profitable basis, if at all.

In addition, because our gene regulation technology is still in the research stage, we have not yet been able to assess safety in humans, and there may be long-term effects from treatment that we cannot predict at this time.

Because gene therapy is novel and the regulatory landscape that governs any product candidates we may develop is uncertain and may change, we cannot predict the time and cost of obtaining regulatory approval, if we receive it at all, for any product candidates we may develop.

The regulatory requirements that will govern any novel gene therapy product candidates we develop are not entirely clear and may change. Within the broader genetic medicine field, a limited number of therapeutic products have received approval from the FDA or an MA from the MHRA and European Commission. Even with respect to more established products that fit into the categories of gene therapies or cell therapies, the regulatory landscape is still developing. Regulatory requirements governing gene therapy products and cell therapy products have changed frequently and will likely continue to change in the future. Moreover, there is substantial, and sometimes uncoordinated, overlap in those responsible for regulation of existing gene therapy products and cell therapy products, which could impact the timing and cost of any regulatory approval. For example, in the United States, the FDA has established the Office of Therapeutic Products within its Center for Biologics Evaluation and Research, or CBER, to consolidate the review of gene therapy and related products, and the Cellular, Tissue and Gene Therapies Advisory Committee to advise CBER on its review. Gene therapy clinical trials may also be subject to review and oversight by an institutional biosafety committee and/or an institutional review board, or IRB, which are local institutional committees or boards, as applicable, that review, approve and oversee basic and clinical research conducted at the institution participating in the clinical trial.

In the EU, the EMA's Committee for Advanced Therapies, or CAT, is responsible for assessing the quality, safety, and efficacy of advanced therapy medicinal products, or ATMPs. ATMPs include gene therapy medicines, somatic-cell therapy medicines and tissue-engineered medicines. The role of the CAT is to prepare a draft opinion on an application for MA for a gene therapy medicinal candidate that is submitted to the EMA. In the EU, the development and evaluation of a gene therapy product must be considered in the context of the relevant EU guidelines. The EMA may issue new guidelines concerning the development and MA for gene therapy products and require that we comply with these new guidelines. As a result, the procedures and standards applied to gene therapy products and cell therapy products may be applied to any gene therapy product candidate we may develop, but that remains uncertain at this point.

Post Brexit, marketing authorization applications, or MAAs, for ATMPs in the UK are regulated nationally and assessed in accordance with the general provisions in place for the licensing of medicines, taking the specific requirements for this group of medicines into account. Definitions for individual classes of ATMPs remain unchanged and classification of ATMPs are undertaken by the MHRA. Data, traceability, exemptions from licensing, packaging and post-authorization requirements remain in line with EU requirements transposed into UK law. However, if the EMA issues new guidance on ATMPs going forward, there is a risk of regulatory divergence with the MHRA and separate procedures and standards with which we may need to comply.

Adverse developments in preclinical studies or clinical trials conducted by others in the field of gene therapy and gene regulation products may cause the FDA, MHRA and other regulatory bodies to revise the requirements for approval of any product candidates we may develop or limit the use of products utilizing gene regulation technologies, either of which could harm our business. In addition, the clinical trial requirements of the FDA, MHRA and other regulatory authorities and the criteria these regulators use to determine the safety and efficacy of a product candidate vary substantially according to the type, complexity, novelty, and intended use and market of the potential products. The regulatory approval process for product candidates such as ours can be more expensive and take longer than for other, better known, or more extensively studied pharmaceutical or other product candidates. Further, as we are developing novel treatments for diseases or conditions in which there may be limited clinical experience with novel endpoints and methodologies, there is heightened risk that the FDA, MHRA, EMA or other regulatory bodies may not consider the clinical trial endpoints we pursue to provide clinically meaningful results, and the resulting clinical data and results may be more difficult to analyze. The prospectively designed natural history studies with the same endpoints as our corresponding clinical trials may not be accepted by the FDA, MHRA, EMA or other regulatory authorities. Regulatory agencies administering existing or future regulations or legislation may not allow production and marketing of products utilizing gene regulation technology in a timely manner or under technically or commercially feasible conditions. In addition, regulatory action or private litigation could result in expenses, delays, or other impediments to our research programs or the commercialization of resulting products.

The regulatory review committees and advisory groups described above and the new guidelines they promulgate may lengthen the regulatory review process, require us to perform additional preclinical studies or clinical trials, increase our development costs, lead to changes in regulatory positions and interpretations, delay or prevent approval and commercialization of these treatment candidates, or lead to significant post-approval limitations or restrictions. As we advance our research programs and develop future product candidates, we will be required to consult with these regulatory and advisory groups and to comply with applicable guidelines. If we fail to do so, we may be required to delay or discontinue development of any product candidates we identify and develop.

Clinical trials are expensive, time-consuming, difficult to design and implement, and involve an uncertain outcome. Further, we may encounter substantial delays in our clinical trials.

The clinical trials and manufacturing of our product candidates are, and the manufacturing and marketing of our products, if approved, will be, subject to extensive and rigorous review and regulation by numerous government authorities in the United States and in other countries where we intend to test and market our product candidates. Before obtaining regulatory approvals for the commercial sale of any of our product candidates, we must demonstrate through lengthy, complex and expensive preclinical testing and clinical trials that our product candidates are both safe and effective for use in each target indication. In particular, because our product candidates are subject to regulation as biological drug products, we will need to demonstrate that they are safe, pure, and potent for use in their target

indications. Each product candidate must demonstrate an adequate risk versus benefit profile in its intended patient population and for its intended use.

Clinical testing is expensive, can take many years to complete and is subject to uncertainty. We cannot guarantee that any clinical trials will be conducted as planned or completed on schedule, if at all. Failure can occur at any time during the clinical trial process. Even if our future clinical trials are completed as planned, we cannot be certain that their results will support the safety and effectiveness of our product candidates for their targeted indications. Our future clinical trial results may not be successful.

In addition, even if such trials are successfully completed, we cannot guarantee that the FDA, MHRA, EMA or other regulatory authorities will interpret the results as we do, and more trials could be required before we submit our product candidates for approval. To the extent that the results of the trials are not satisfactory to the FDA, MHRA, EMA or other regulatory authorities for support of an MAA, we may be required to expend significant resources, which may not be available to us, to conduct additional trials in support of potential approval of our product candidates.

To date, we have not obtained regulatory approval for any of our product candidates, and additional clinical development may be required before we are able to obtain any such approval. Although we are currently conducting several clinical development programs, we may experience delays in conducting any clinical trials and we do not know whether our ongoing and future clinical trials will begin on time, need to be redesigned, be able to recruit and enroll patients on time or be completed on schedule, or at all. Events that may prevent successful or timely completion of clinical development include:

- inability to generate sufficient preclinical, toxicology, or other *in vivo* or *in vitro* data to support the initiation of clinical trials;
- delays in sufficiently developing, characterizing or controlling a manufacturing process suitable for advanced clinical trials;
- delays in developing suitable assays for screening patients for eligibility for trials with respect to certain product candidates;
- delays in reaching consensus with the FDA, MHRA or other regulatory authorities as to the design or implementation of our clinical trials and obtaining regulatory allowance or approval to commence a clinical trial;
- inability to reach an agreement on acceptable terms with clinical trial sites or prospective contract research organizations, or CROs, the terms of which can be subject to extensive negotiation and may vary significantly among different clinical trial sites;
- our inability to recruit and train clinical trial investigators with the appropriate competencies and experience to conduct the clinical trials, administer our product candidates and oversee clinical trial staff;
- delays in obtaining IRB or ethics committee approval or positive opinion at each site;
- inability to recruit suitable patients to participate in a clinical trial;
- inability to develop and validate any companion diagnostic we may decide to use in connection with a clinical trial, if applicable;
- delays in sufficiently developing, designing and manufacturing equipment or medical devices used to administer our product candidates in our clinical trials, if applicable;

- patients not completing a clinical trial or returning for post-treatment follow-up;
- clinical sites, CROs, or other third parties deviating from trial protocol or dropping out of a trial;
- failures to conduct clinical trials in accordance with good clinical practice, or GCP, requirements, or other applicable regulatory guidelines;
- addressing patient safety concerns that arise during the course of a trial, including occurrence of adverse events associated with the product candidate;
- having an insufficient number of clinical trial sites; or
- inability to manufacture sufficient quantities of our product candidates for use in clinical trials, or to manufacture such product candidates to acceptable quality standards.

We may experience numerous unforeseen events during, or as a result of, clinical trials that could delay or prevent our ability to receive marketing approval or commercialize our product candidates or significantly increase the cost of such trials, including:

- we may experience changes in regulatory requirements or guidance, or receive feedback from regulatory authorities that requires us to modify the design of our clinical trials;
- clinical trials of our product candidates may produce negative or inconclusive results, and we may decide, or regulators may require us, to conduct additional clinical trials or abandon development programs;
- the number of patients required for clinical trials of our product candidates may be larger than we anticipate, enrollment in these clinical trials may be slower than we anticipate, or participants may drop out of these clinical trials at a higher rate than we anticipate;
- our third-party contractors may fail to comply with regulatory requirements or meet their contractual obligations to us in a timely manner, or at all;
- we or our investigators might have to suspend or terminate clinical trials of our product candidates for various reasons, including non-compliance with regulatory requirements, a finding that our product candidates have undesirable side effects or other unexpected characteristics, or a finding that the participants are being exposed to unacceptable health risks;
- the cost of clinical trials of our product candidates may be greater than we anticipate, and we may not have funds to cover the costs;
- the supply or quality of our product candidates or other materials necessary to conduct clinical trials of our product candidates may be insufficient or inadequate;
- business interruptions resulting from geopolitical actions, including war and terrorism, or a widespread health emergency, or natural disasters including earthquakes, typhoons, floods and fires, or from economic or political instability; and
- any future collaborators that conduct clinical trials may face any of the above issues, and they may conduct clinical trials in ways they view as advantageous to them but that are suboptimal for us.

If we are required to conduct additional clinical trials or other testing of our product candidates beyond those that we currently contemplate, if we are unable to successfully complete clinical trials of our product candidates or other testing, if the results of these trials or tests are not positive or are only modestly positive or if there are safety concerns, we may:

- incur unplanned costs;
- be delayed in obtaining marketing approval for our product candidates or not obtain marketing approval at all;
- obtain marketing approval in some countries and not in others;
- obtain marketing approval for indications or patient populations that are not as broad as intended or desired;
- obtain marketing approval with labeling that includes significant use or distribution restrictions or safety warnings, including boxed warnings;
- be subject to additional post-marketing testing requirements; or
- have the product removed from the market after obtaining marketing approval.

We could encounter delays if a clinical trial is suspended or terminated by us, by the IRBs of the institutions in which such trials are being conducted, by the Data Safety Monitoring Board, or DSMB, for such trial or by the FDA, MHRA or other foreign regulatory authorities. Such authorities may impose such a suspension or termination due to a number of factors, including failure to conduct the clinical trial in accordance with regulatory requirements or our clinical protocols, inspection of the clinical trial operations or trial site by the FDA, MHRA or other regulatory authorities resulting in the imposition of a clinical hold, unforeseen safety issues or adverse side effects, failure to demonstrate a benefit from using a drug, changes in governmental regulations or administrative actions or lack of adequate funding to continue the clinical trial.

Our product candidates will require extensive clinical testing before we are prepared to submit a BLA or MAA for regulatory approval. We cannot predict with any certainty if or when we might complete the clinical development for our product candidates and submit a BLA or MAA for regulatory approval of any of our product candidates or whether any such BLA or MAA will be approved. We may also seek feedback from the FDA, MHRA, EMA or other regulatory authorities on our clinical development program, and the FDA, MHRA, EMA or such regulatory authorities may not provide such feedback on a timely basis, or such feedback may not be favorable, which could further delay our development programs.

If we experience delays in the commencement or completion of our clinical trials, or if we terminate a clinical trial prior to completion, the commercial prospects of our product candidates could be harmed, and our ability to generate revenues from our product candidates may be delayed. In addition, any delays in our clinical trials could increase our costs, slow down the development and approval process and jeopardize our ability to commence product sales and generate revenues. Any of these occurrences may harm our business, financial condition and results of operations. In addition, many of the factors that cause, or lead to, a delay in the commencement or completion of clinical trials may also ultimately lead to the denial of regulatory approval of our product candidates. For example changes in the leadership of the FDA and other federal agencies under the current presidential administration or reductions in funding, operations, staffing and policies of the FDA and other federal agencies could impact our clinical development plans and timelines.

In addition, the FDA's and other regulatory authorities' policies with respect to clinical trials may change and additional government regulations may be enacted. For instance, the regulatory landscape related to clinical trials in the EU has evolved over recent years. As of January 2025, clinical trials (and related applications) in the EU are now fully subject to the provisions of the EU Clinical Trials Regulation, or CTR, which allows sponsors to make a single submission to both the competent authority and an ethics committee in each member state, leading to a single decision per member state and provides for a joint assessment by all member states concerned, and a separate assessment by each member state with respect to specific requirements related to its own territory, including ethics rules. Each member state's decision is communicated to the sponsor via the centralized EU portal. Once the CTA is approved, clinical study development may proceed. Compliance with the CTR requirements by us and our third-party service providers, such as CROs, may impact our development plans.

Furthermore, on April 28, 2025, the UK adopted amendments to the Medicines for Human Use (Clinical Trials) Regulations 2004 intended to support a more streamlined and flexible regulation of clinical trials, removing unnecessary administrative burdens on trial sponsors, while protecting the interests of trial participants. It also intends to bring the UK regulatory framework for clinical trials, which is still based on the EU Clinical Trials Directive, into closer alignment with the CTR. The amendment became applicable on April 28, 2026, following a one-year transition period, and the MHRA published guidance to support stakeholders during the transition period and has published, and may continue to publish, further guidance regarding implementation of the amended framework.

If we are slow or unable to adapt to changes in existing requirements or the adoption of new requirements or policies governing clinical trials, our development plans may also be impacted.

Pandemics, epidemics or outbreaks of an infectious disease have impacted and may in the future materially and adversely impact our business, including our preclinical studies, clinical trials, manufacturing capabilities and regulatory approvals.

We have and may in the future experience disruptions from pandemics, epidemics or outbreaks of an infectious disease that could severely impact our business, preclinical studies, clinical trials and laboratory and manufacturing activities, including, for example, delays or difficulties in enrolling patients in our clinical trials, delays or difficulties in clinical site initiation, including difficulties in recruiting clinical site investigators and clinical site staff, diversion of healthcare resources away from the conduct of clinical trials, interruption of key clinical trial activities due to limitations on travel imposed or recommended by regulatory authorities or others, interruption or delays in the operations of the FDA, MHRA, EMA or other regulatory authorities, interruption of, or delays in, the manufacturing of our product candidates, interruptions in preclinical studies due to restricted or limited operations at our laboratory facilities, limitations on employee resources that would otherwise be focused on the conduct of our preclinical studies and clinical trials, and interruption or delays to our sourced discovery and clinical activities. For example, as a result of the COVID-19 pandemic, we restricted onsite activities and also experienced some delays in enrolling, treating and monitoring patients in our clinical trials, as well as limited disruptions to our supply chain.

The extent to which any future outbreaks or any variants of COVID-19 or another pandemic may impact our business, preclinical studies, clinical trials and laboratory and manufacturing activities will depend on future developments, which are highly uncertain and cannot be predicted with confidence, such as the duration of any pandemic, the timing, distribution and effectiveness of vaccines, vaccination rates, travel restrictions and physical distancing requirements in the countries where we do business, business closures or business disruptions, and the effectiveness of actions taken in the countries where we do business to contain and treat any such disease, respond to the reduction in global economic activity and resume normal economic and operating conditions. If we or any of the third parties with whom we engage experience prolonged shutdowns or other business disruptions, our ability to conduct our business in the manner and on the timelines presently planned could be materially and negatively impacted. Furthermore, the magnitude of the economic impact of any pandemic including sustained inflation, supply chain disruptions, and major central bank policy actions may result in significant disruption of global financial markets, which could materially affect our performance, financial condition, results of operations, and cash flows, as well as our ability to raise additional capital.

The affected populations for our product candidates may be smaller than we or third parties currently project, which may affect the addressable markets for our product candidates.

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 product candidates, are estimates based on our knowledge and understanding of these diseases. The total addressable market opportunity for our product candidates will ultimately depend upon a number of factors including the diagnosis and treatment criteria included in the final label, if approved for sale in specified indications, acceptance by the medical community, patient access and product pricing and reimbursement. Incidence and prevalence estimates are frequently based on information and assumptions that are not exact and may not be appropriate, and the methodology is forward-looking and speculative. The process we have used in developing an estimated incidence and prevalence range for the indications we are targeting has involved collating limited data from multiple sources. Accordingly, the incidence and prevalence estimates included, or supporting the information, in our SEC filings and other materials should be viewed with caution. Further, the data and statistical information included, or supporting the information, in our SEC filings and other materials, including estimates derived from them, may differ from information and estimates made by our competitors or from current or future studies conducted by independent sources.

The use of such data involves risks and uncertainties and is subject to change based on various factors. Our estimates may prove to be incorrect and new studies may change the estimated incidence or prevalence of the diseases we seek to address. The number of patients with the diseases we are targeting in the United States, the UK, the EU and elsewhere may turn out to be lower than expected or may not be otherwise amenable to treatment with our products, or new patients may become increasingly difficult to identify or access, all of which would harm our results of operations and our business.

Negative public opinion of gene therapy and increased regulatory scrutiny of gene therapy and genetic research may adversely impact public perception of our current and future product candidates.

Our potential therapeutic products involve introducing genetic material into patients' cells. The clinical and commercial success of our potential products will depend in part on public acceptance of the use of gene therapy and gene regulation for the prevention or treatment of human diseases. Public attitudes may be influenced by claims that gene therapy and gene regulation are unsafe, unethical, or immoral, and, consequently, our products may not gain the acceptance of the public or the medical community. Public attitudes may adversely impact our ability to enroll clinical trials. Moreover, our success will depend upon physicians prescribing, and their patients being willing to receive, treatments that involve the use of product candidates we may develop in lieu of, or in addition to, existing treatments with which they are already familiar and for which greater clinical data may be available.

More restrictive government regulations or negative public opinion would have a negative effect on our business or financial condition and may delay or impair the development and commercialization of our product candidates or demand for any products once approved. Additionally, adverse events in our clinical trials, even if not ultimately attributable to our product candidates, and the resulting publicity could result in increased governmental regulation, unfavorable public perception, potential regulatory delays in the testing or approval of our product candidates or the halting of clinical trials, stricter labeling requirements for those product candidates that are approved and a decrease in demand for any such product candidates. The risk of cancer remains a concern for gene therapy and we cannot assure that it will not occur in any of our planned or future clinical trials. 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. If any such adverse events occur, commercialization of our product candidates or further advancement of our clinical trials could be halted or delayed, which would have a negative impact on our business and operations.

We may fail to maintain the benefits of certain regulatory designations that we have obtained for our product candidates, and may in the future seek and fail to obtain such designations for other of our current or potential future product candidates. Even if such designations are obtained, they may not lead to faster development or regulatory review or approval, and they do not increase the likelihood that our product candidates will receive marketing approval.

A sponsor may seek approval of its product candidate under programs designed to accelerate the FDA's review and approval of drugs and biological products that meet certain criteria. For example, the FDA has a Fast Track designation program that is intended to expedite or facilitate the process for reviewing product candidates that meet certain criteria. Specifically, investigational drugs and biological products are eligible for Fast Track designation if they are intended to treat a serious condition and nonclinical or clinical data demonstrate the potential to address unmet medical needs. Fast Track designation applies to the combination of the product candidate and the specific indication for which it is being studied. The sponsor of a Fast Track product candidate has opportunities for more frequent interactions with the review team during product development and, once a BLA is submitted, the application may be eligible for priority review. In addition, the Fast Track product may be eligible for rolling review, where the FDA may consider for review sections of the BLA on a rolling basis before the complete application is submitted if the sponsor provides a schedule for the submission of the sections of the application, the FDA agrees to accept sections of the application and determines that the schedule is acceptable, and the sponsor pays any required user fees upon submission of the first section of the application. Even if Fast Track designation is granted, it may be rescinded if the product no longer meets the qualifying criteria. In April 2018, bota-vec was issued Fast Track Designation by the FDA for the treatment of X-linked retinitis pigmentosa owing to defects in RPGR. In August 2018, AAV-CNGB3 was issued Fast Track designation by the FDA for the treatment of achromatopsia caused by CNGB3 mutations. In January 2021, AAV-CNGA3 was issued Fast Track designation by the FDA for the treatment of achromatopsia caused by CNGA3 mutations.

Similarly, the EMA has established the PRIME scheme to expedite the development and review of product candidates that show a potential to address to a significant extent an unmet medical need, based on early clinical data. In February 2018, AAV-CNGB3 for the treatment of achromatopsia associated with defects in CNGB3 was admitted to the PRIME scheme of the EMA. In February 2020, bota-vec for the treatment of X-linked retinitis pigmentosa owing to defects in RPGR was admitted to the PRIME scheme of the EMA.

A sponsor may also seek a Regenerative Medicine Advanced Therapy, or RMAT, designation for its product candidates. In 2017, the FDA established the RMAT designation as part of its implementation of the 21st Century Cures Act. A biological product is eligible for RMAT designation if it qualifies as an RMAT, which is defined as a cell therapy, therapeutic tissue engineering product, human cell and tissue product, or any combination product using such therapies or products, with limited exceptions, and is intended to treat, modify, reverse, or cure a serious or life-threatening disease or condition and for which preliminary clinical evidence indicates that the biological product has the potential to address unmet medical needs for such a disease or condition. In a February 2019 guidance, the FDA also stated that certain gene therapies that lead to a sustained effect on cells or tissues may meet the definition of a regenerative medicine therapy. RMAT designation provides potential benefits that include more frequent meetings with the FDA to discuss the development plan for the product candidate, and eligibility for rolling review and priority review, provided the applicable criteria are met. Products granted RMAT designation may also be eligible for accelerated approval on the basis of a surrogate or intermediate endpoint reasonably likely to predict long-term clinical benefit, or reliance upon data obtained from a meaningful number of sites, including through expansion to additional sites. RMAT-designated products that receive accelerated approval may, as appropriate, fulfill their post-approval requirements through the submission of clinical evidence, clinical trials, patient registries, or other sources of real world evidence (such as electronic health records); through the collection of larger confirmatory data sets; or via post-approval monitoring of all patients treated with such therapy prior to approval of the therapy. In December 2024, the FDA granted RMAT designation to AAV-hAQP1 for the treatment of grade 2 or 3 radiation-induced xerostomia, and in March 2026, the FDA granted Breakthrough Therapy Designation for the same program.

In May 2025, the FDA granted RMAT designation to AAV-GAD for the treatment of Parkinson's disease not adequately controlled with anti-Parkinsonian medications.

Such regulatory designations are within the discretion of the FDA, MHRA, EMA and other regulatory authorities. Accordingly, even if we believe one of our product candidates meets the criteria for such regulatory programs and we seek such designations, the FDA, MHRA, EMA or other applicable regulatory authority may disagree and instead determine not to make such designation for such product candidate. We cannot be sure that our evaluation of our product candidates as qualifying for such regulatory designations will meet the regulatory authority's expectations. In any event, the receipt of such regulatory designations for a product candidate may not result in a faster development process, review, or approval compared to product candidates considered for approval under conventional regulatory procedures and does not assure ultimate approval by the regulatory authorities. In addition, even if additional product candidates are granted such regulatory designations, the regulatory authority may later decide that such product candidates no longer meet the conditions for qualification or decide that the time period for review or approval will not be shortened, as applicable.

We have received orphan drug designation and orphan designation from the FDA and European Commission, respectively, for bota-vec, AAV-AIPL1, AAV-CNGB3, AAV-CNGA3, AAV-RPE65, AAV-RDH12 and orphan drug designation from the FDA for AAV-hAQP1 and AAV-BBS10, and we may seek orphan drug designation or orphan designation for additional product candidates in the future, but any orphan drug designations or orphan designations we have received or may receive in the future may not confer marketing exclusivity or other expected benefits.

Under the Orphan Drug Act, the FDA may designate a product candidate as an orphan drug if it is intended to treat a rare disease or condition, defined as one occurring in a patient population of fewer than 200,000 in the United States, or a patient population greater than 200,000 in the United States where there is no reasonable expectation that the cost of developing the drug will be recovered from sales in the United States. In the EU, the European Commission grants orphan designation on the basis of the EMA's Committee for Orphan Medicinal Products opinion. A medicinal product may be designated as orphan if (1) it is intended for the diagnosis, prevention or treatment of a life-threatening or chronically debilitating condition; (2) either (a) such condition affects no more than five in 10,000 persons in the EU when the application is made, or (b) the product, without the benefits derived from orphan status, would not generate sufficient return in the EU to justify investment; and (3) there exists no satisfactory method of diagnosis, prevention or treatment, of such condition authorized for marketing in the EU, or if such a method exists, the product will be of significant benefit to those affected by the condition.

In the United States, orphan drug designation entitles a party to financial incentives such as opportunities for grant funding towards clinical trial costs, tax credits for qualified clinical testing, and user-fee waivers. In addition, if a product receives the first FDA approval of that drug for the disease or condition for which it has orphan drug designation, the product is entitled to orphan drug exclusivity, which means the FDA may not approve any other application to market the same drug for the same approved use or condition for a period of seven years, except in limited circumstances, such as a showing of clinical superiority over the product with orphan exclusivity or where the manufacturer is unable to assure the availability of sufficient quantities of the orphan drug to meet the needs of patients with the rare disease or condition. In the EU, orphan designation entitles a party to financial incentives such as reduction of fees or fee waivers, protocol assistance, and access to the centralized MA procedure. Moreover, upon grant of an MA and assuming the requirement for orphan designation are also met at the time the MA is granted, orphan medicinal products are entitled to a ten-year period of market exclusivity for the approved therapeutic indication. The period of market exclusivity is extended by two years for orphan medicinal products that have also complied with an agreed pediatric investigation plan, or PIP. This period may be reduced to six years if, at the end of the fifth year, the orphan designation criteria are no longer met, including where it is shown that the product is sufficiently profitable not to justify maintenance of market exclusivity, or where the prevalence of the condition has increased above the orphan designation threshold. In the EU, an MA for an orphan designated product will not be granted if a similar product has been approved in the EU for the same therapeutic indication, unless the applicant can establish that (i) its product, although similar to the orphan medicinal product already authorized is safer, more effective or otherwise clinically superior; (ii) the MA holder for the orphan medicinal product grants its consent; or (iii) if the MA holder of the orphan medicinal product is unable to supply sufficient quantities of product. A similar medicine is a product containing a similar active substance or substances as those contained in an already authorized product. Similar active substance is defined as an identical active

substance, or an active substance with the same principal molecular structural features (but not necessarily all of the same molecular features) and which acts via the same mechanism.

There is no pre-MA orphan designation in the UK. The MHRA reviews applications from companies for orphan designation in parallel to the corresponding MAA. The criteria are essentially the same, but have been tailored for the market, i.e., the prevalence of the condition in the UK, rather than the EU, must not be more than five in 10,000. Should an orphan designation be granted, the period or market exclusivity will be set from the date of first approval of the product in the UK.

We have obtained orphan drug designation from the FDA and orphan designation from the European Commission for botavec for the treatment of X-linked retinitis pigmentosa associated with mutations in the RPGR gene, for AAV-AIPL1 for the treatment of inherited retinal dystrophy due to defects in AIPL1 gene, for AAV-CNGB3 for the treatment of achromatopsia caused by mutations in the *CNGB3* gene, for AAV-CNGA3 for the treatment of achromatopsia due to autosomal-recessive *CNGA3* gene mutations, for AAV-RPE65 for the treatment of Leber congenital amaurosis and for AAV-RDH12 for the treatment of retinol dehydrogenase 12 (RDH12) mutation-associated retinal dystrophy, and we obtained orphan drug designation from the FDA for AAV-hAQP1 for the treatment of grade 2 and grade 3 late xerostomia from parotid gland hypofunction caused by radiotherapy and for AAV-BBS10 for the treatment of Bardet-Biedel syndrome (BBS) due to *BBS10* mutations. We may seek orphan drug designation and orphan designation for other current and future product candidates. Even with orphan drug designation and orphan designation, we may not be the first to obtain marketing approval for any particular orphan indication due to the uncertainties associated with developing pharmaceutical products, which could prevent us from marketing our product candidates if another company is able to obtain orphan drug exclusivity before we do. In addition, exclusive marketing rights in the United States and the EU may be unavailable if we seek approval for a disease or condition broader than the orphan drug-designated and orphan-designated disease or condition or may be lost in the United States or EU if the FDA or foreign authorities later determine that the request for designation was materially defective or if we are unable to assure sufficient quantities of the drug to meet the needs of patients with the rare disease or condition following approval.

Further, even if we obtain orphan drug exclusivity, that exclusivity may not effectively protect our product candidates from competition because different biologics with different active principal molecular structural features can be approved for the same disease or condition. In addition, the FDA can subsequently approve products with the same principal molecular structural features, in the case of a biologic, for the same use or indication if the FDA concludes that the later product is safer, more effective, makes a major contribution to patient care, or if the manufacturer of the product with orphan exclusivity is unable to maintain sufficient product quantity. Likewise, in the EU and UK, the European Commission or MHRA, respectively, can authorize a similar product for the same therapeutic indication, if it concludes that the later product is safer, more effective or clinically superior; if the MA holder for the initial orphan medicinal product grants its consent; or if such MA holder is unable to supply sufficient quantities of the product. Neither orphan drug designation nor orphan designation shortens the development time or regulatory review time of a drug nor gives the drug any advantage in the regulatory review or approval process. In addition, while we intend to seek orphan drug designation and orphan designation for other existing and future product candidates, we may never receive such designations.

The FDA has granted rare pediatric disease designation to a number of our gene therapy product candidates, however, there is no guarantee that FDA approval of any of these gene therapy candidates will result in a priority review voucher.

In 2012, Congress authorized the FDA to award priority review vouchers to sponsors of certain rare pediatric disease drugs and biologics intended to treat certain orphan diseases affecting fewer than 200,000 patients in the U.S., the serious or life-threatening manifestations of which primarily affect individuals aged 18 years of age or younger, or affects more than 200,000 individuals in the U.S. and for which there is no reasonable expectation that the cost of developing and making available in the U.S. a drug for such disease or condition will be recovered from sales in the U.S. of such drug. This program is designed to encourage development of new drug and biological products for prevention and treatment of certain rare pediatric diseases. Specifically, under this program, a sponsor who receives an approval for a drug or biologic for a “rare pediatric disease” that meets certain criteria may be eligible to receive a voucher that can be redeemed to receive a priority review of a subsequent marketing application for a different product. The sponsor of a

rare pediatric disease drug product receiving a priority review voucher may transfer (including by sale) the voucher to another sponsor, and such priority review vouchers have recently sold for between $150 million to $200 million. The voucher may be further transferred any number of times before the voucher is used, as long as the sponsor making the transfer has not yet submitted the application. The FDA may also revoke any priority review voucher if the rare pediatric disease drug for which the voucher was awarded is not marketed in the U.S. within one year following the date of approval.

The FDA has granted us rare pediatric disease designation for AAV-AIPL1 for the treatment of Leber congenital amaurosis (LCA4) retinal dystrophy, AAV-BBS10 for the treatment of BBS due to *BBS10* mutations, AAV-RDH12 for the treatment of Leber congenital amaurosis (LCA) and early-onset severe retinal dystrophy (EOSRD), AAV8-RK-RetGC for the treatment of patients with Leber congenital amaurosis due to *GUCY2D* mutations (LCA1), AAV-RPE65 for the treatment of inherited retinal dystrophy due to biallelic *RPE65* mutations, AAV-CNGB3 for the treatment of achromatopsia caused by mutations in the *CNGB3* gene and AAV-CNGA3 for the treatment of achromatopsia caused by mutations in the *CNGA3* gene. There is no guarantee that we will be able to obtain a priority review voucher, even if one or more of these gene therapy product candidates is approved by the FDA. While the rare pediatric disease priority voucher program began to sunset on December 20, 2024, the Consolidated Appropriations Act of 2026 signed into law on February 3, 2026, extended the program through September 30, 2029. Therefore, the sponsor of the marketing application for a drug that receives rare pediatric disease designation will be eligible to receive a voucher if the FDA approves the product for use within the designated rare pediatric disease on or before September 30, 2029, unless Congress reauthorizes the program further.

We and our contract manufacturers for plasmid are subject to significant regulation with respect to manufacturing our products. Our manufacturing facilities and the third-party manufacturing facilities which we rely on may not continue to meet regulatory requirements and have limited capacity.

We currently have relationships with a limited number of suppliers for the manufacturing of plasmid, a component of our viral vectors and product candidates. We also have GMP manufacturing facilities in London, United Kingdom and Shannon, Ireland, which we expect can supply our current clinical and preclinical programs, as well as any anticipated third party supply obligations, through regulatory approval and, should they be approved, provide sufficient capacity for their commercial production. However, if we experience slowdowns or problems with our facilities or are unable to establish or scale our internal manufacturing capabilities, we will need to continue to contract with manufacturers that can produce the preclinical, clinical and commercial supply of our products. Each supplier may require licenses to manufacture such components if such processes are not owned by the supplier or in the public domain and we may be unable to transfer or sublicense the intellectual property rights we may have with respect to such activities.

All entities involved in the preparation of therapeutics for clinical trials or commercial sale, including our existing contract manufacturers for components of our product candidates, are subject to extensive regulation. Components of a finished therapeutic product approved for commercial sale or used in late-stage clinical trials must be manufactured in accordance with GMP. These regulations govern manufacturing processes and procedures (including record keeping) and the implementation and operation of quality systems to control and assure the quality of investigational products and products approved for sale. Poor control of production processes can lead to the introduction of adventitious agents or other contaminants, or to inadvertent changes in the properties or stability of our product candidates that may not be detectable in final product testing. We or our contract manufacturers must supply all necessary documentation in support of a BLA or MAA on a timely basis. Generally, our facilities and quality systems and the facilities and quality systems of some or all of our third-party contractors must successfully complete a pre-approval inspection for compliance with the applicable regulations as a condition of regulatory approval of our product candidates or the product candidates that we manufacture for third parties. In addition, certain regulatory authorities may, at any time, audit or inspect a manufacturing facility involved with the preparation of our product candidates or the product candidates that we manufacture for third parties or the associated quality systems for compliance with the regulations applicable, if and when approved, to the activities being conducted. If these facilities do not successfully complete a pre-approval plant inspection, FDA, MHRA or other regulatory approval of the product candidates will not be granted.

If any such inspection or audit identifies a failure to comply with applicable regulations or if a violation of our product specifications or applicable regulations occurs independent of such an inspection or audit, we or the relevant regulatory authority may require remedial measures that may be costly and/or time-consuming for us or a third party to implement and that may include the temporary or permanent suspension of a clinical trial or commercial sales or the temporary or permanent closure of a facility. Any such remedial measures imposed upon us or third parties with whom we contract could harm our business. If we or any of our third-party manufacturers fail to maintain regulatory compliance, the FDA, MHRA or other regulatory authorities can impose regulatory sanctions including, among other things, refusal to approve a pending application for a new drug product or biologic product, or revocation of a pre-existing approval. As a result, our business, financial condition and results of operations may be harmed. Additionally, if supply from one approved manufacturer is interrupted, there could be a significant disruption in commercial supply. An alternative manufacturer would need to be qualified through a BLA and/or MAA supplement which could result in further delay. Regulatory agencies may also require additional studies if a new manufacturer is relied upon for clinical or commercial production. Switching manufacturers may involve substantial costs and is likely to result in a delay in our desired clinical and commercial timelines.

These factors could cause the delay of clinical trials, regulatory submissions, required approvals or commercialization of our product candidates, cause us to incur higher costs and prevent us from commercializing our products successfully. Furthermore, if our suppliers fail to meet contractual requirements, and we are unable to secure one or more replacement suppliers capable of production at a substantially equivalent cost, our clinical trials may be delayed, or we could lose potential revenue.

Any contamination in our manufacturing process, shortages of raw materials or failure of our plasmid supplier to deliver necessary components, or other issues with the manufacturing process, could result in delays in our clinical development or marketing schedules.

Given the nature of biologics manufacturing, there is a risk of contamination. Any contamination could adversely affect our ability to produce product candidates on schedule and could, therefore, harm our results of operations and cause reputational damage. Some of the raw materials required in our manufacturing process are derived from biologic sources. Such raw materials are difficult to procure and may be subject to contamination or recall. In addition, our manufacturing process is complex, and the manufacturing batch cycle period can be several weeks long. Each batch cycle may not yield planned quantities or meet the required standards. A material shortage, contamination, recall or restriction on the use of biologically derived substances in the manufacture of our product candidates, failure of manufacturing equipment or systems or other issues with our manufacturing process, could adversely impact or disrupt the commercial manufacturing or the production of clinical material, which could adversely affect our development timelines and our business, financial condition, results of operations and prospects.

Expanding our manufacturing capacity has and will continue to be costly and we may be unsuccessful in doing so in a timely manner, which could delay our current and future clinical development programs, or delay the commercialization of our product candidates.

In addition to our existing manufacturing facilities in London, United Kingdom and Shannon, Ireland, we may lease, operate, purchase, or construct additional facilities to supply our clinical and preclinical programs, as well as to meet any anticipated third party supply obligations, or conduct expanded manufacturing or other related activities in the future. Expanding our manufacturing capacity to produce the preclinical, clinical and commercial supply of our products and their components has required and will continue to require substantial additional expenditures, time, and various regulatory approvals and permits, as well as hiring, training and retraining employees and managerial personnel to staff our manufacturing and supply chain operations. Start-up costs can be large and may exceed our expectations, and scale-up entails significant risks related to process development and manufacturing yields. In addition, we may face difficulties or delays in developing or acquiring the necessary production equipment and technology to manufacture sufficient quantities of our product candidates for use in clinical trials and, should they be approved, to supply the commercial market at reasonable costs and in compliance with applicable regulatory requirements. We may not successfully expand, establish or sustain sufficient manufacturing capabilities or manufacture our products economically or in compliance with GMP and other regulatory requirements, and we and our collaborators may not be able to build or procure additional capacity in the required timeframe to meet the requirements of our clinical programs or to meet

potential commercial demand for our product candidates. This could also delay or require us to discontinue one or more of our clinical development programs or could interfere with our efforts to successfully commercialize our products. As a result, our business, prospects, operating results, and financial condition could be materially harmed.

If we encounter difficulties enrolling patients in our clinical trials, our clinical development activities could be delayed or otherwise adversely affected.

The timely completion of clinical trials in accordance with their protocols depends, among other things, on our ability to enroll a sufficient number of patients who remain in the study until its conclusion. We may encounter delays in enrolling, or be unable to enroll, a sufficient number of patients to complete any of our clinical trials, and even once enrolled we may be unable to retain a sufficient number of patients to complete any of our trials. This may result in increased costs, program delays or both, which could have a harmful effect on our ability to develop our product candidates, or could render further development impossible. The enrollment of patients depends on many factors, including:

- the size and nature of the patient population;
- the patient eligibility criteria defined in the protocol;
- the size of the patient population required for analysis of the trial's primary endpoints;
- the proximity of patients to study sites;
- the design of the trial or side effects that may arise in development;
- our ability to recruit clinical trial investigators with the appropriate competencies and experience;
- clinicians' and patients' perceptions as to the potential advantages of the product candidate being studied in relation to other available therapies, including any new products that may be approved for the indications we are investigating;
- our ability to obtain and maintain patient consents;
- the risk that patients enrolled in clinical trials will drop out of the trials before completion; and
- business interruptions resulting from geopolitical actions, including war and terrorism, or widespread health emergencies, or natural disasters including earthquakes, typhoons, floods and fires, or from economic or political instability.

In addition, other clinical trials for product candidates that are in the same therapeutic areas as our product candidates or approved products for the same clinical indications (such as Luxturna marketed by Spark Therapeutics, Inc. for the treatment of RPE65-associated retinal disease) may reduce the number and type of patients available to us. Furthermore, although we have conducted and may in the future conduct natural history studies to better characterize the patient populations we seek to address, any natural history studies we may undertake could fail to provide us with patients for our clinical trials, because patients enrolled in the natural history studies may not be good candidates for our clinical trials or may choose to not enroll in our clinical trials.

Our product candidates may cause serious adverse events or undesirable side effects or have other properties which may delay or prevent their regulatory approval, limit the commercial profile of an approved label, or, result in significant negative consequences following marketing approval, if any.

Serious adverse events or undesirable side effects caused by our product candidates could cause us or regulatory authorities to interrupt, delay or halt clinical trials and could result in a more restrictive label or the delay or denial of regulatory approval by the FDA, MHRA or other authorities. Results of our clinical trials could reveal a high and unacceptable severity and prevalence of side effects, toxicities or unexpected characteristics, including death. A risk in any gene therapy product based on viral vectors is the risk of insertional mutagenesis.

If unacceptable side effects or deaths arise in the development of our product candidates, we, the FDA, the IRBs at the institutions in which our studies are conducted, the DSMB, or other regulatory bodies could suspend or terminate our clinical trials or the FDA, MHRA or other regulatory authorities could order us to cease clinical trials or deny approval of our product candidates for any or all targeted indications. Undesirable side effects or deaths in clinical trials with our product candidates may cause the FDA or comparable foreign regulatory authorities to place a clinical hold on the associated clinical trials, to require additional studies, or otherwise to delay or deny approval of our product candidates for any or all targeted indications. Treatment-related side effects could also affect patient recruitment or the ability of enrolled patients to complete the trial or result in potential product liability claims. In addition, these side effects may not be appropriately recognized or managed by the treating medical staff. We expect to have to train medical personnel using our product candidates to understand the side effect profiles for our clinical trials and upon any commercialization of any of our product candidates. Inadequate training in recognizing or managing the potential side effects of our product candidates could result in patient injury or death. Any of these occurrences may harm our business, financial condition and prospects significantly.

If any of our product candidates receives marketing approval, and we or others later identify undesirable side effects caused by any such product, including during any long-term follow-up observation period recommended or required for patients who receive treatment using our products, a number of potentially significant negative consequences could result, including:

- regulatory authorities may withdraw approvals of such product;
- we may be required to recall a product or change the way such product is administered to patients;
- additional restrictions may be imposed on the marketing of the particular product or the manufacturing processes for the product;
- regulatory authorities may require additional warnings on the label, such as a “black box” warning or contraindication;
- we may be required to implement a Risk Evaluation and Mitigation Strategy, or REMS, or create a medication guide outlining the risks of such side effects for distribution to patients or similar risk management measures;
- the product could become less competitive;
- we could be sued and held liable for harm caused to patients; and
- our reputation may suffer.

Any of these events could prevent us from achieving or maintaining market acceptance of the particular product candidate, if approved, and could significantly harm our business, results of operations and prospects.

Success in preclinical studies or clinical trials may not be indicative of results in future clinical trials.

Results from previous preclinical studies or clinical trials are not necessarily predictive of future clinical trial results, and interim results of a clinical trial are not necessarily indicative of final results. Our product candidates may fail to show the desired safety and efficacy in clinical development despite positive results in preclinical studies or having successfully advanced through initial clinical trials.

Success in preclinical testing and early clinical trials does not ensure that later clinical trials will generate the same results or otherwise provide adequate data to demonstrate the efficacy and safety of a product candidate.

Frequently, product candidates that have shown promising results in early clinical trials have subsequently suffered significant setbacks in later clinical trials. In addition, the design of a clinical trial can determine whether its results will support approval of a product and flaws in the design of a clinical trial may not become apparent until the clinical trial is well advanced. We have limited experience designing clinical trials and may be unable to design and execute a clinical trial to support regulatory approval. There is a high failure rate for drugs and biologic products proceeding through clinical trials. Data obtained from preclinical and clinical activities are subject to varying interpretations, which may delay, limit or prevent regulatory approval, which could negatively impact our business, financial condition, results of operations and prospects.

The regulatory approval processes of the FDA, MHRA, competent authorities in the EU and other regulatory authorities are lengthy, time consuming and inherently unpredictable, and if we are ultimately unable to obtain regulatory approval for our product candidates, our business will be substantially harmed.

The time required to obtain approval by the FDA, MHRA, European Commission and other regulatory authorities is unpredictable but typically takes many years following the commencement of clinical trials and depends upon numerous factors, including the substantial discretion of the regulatory authorities. In addition, approval policies, regulations, or the type and amount of clinical data necessary to gain approval may change during the course of a product candidate's clinical development and may vary among jurisdictions. For example, pharmaceutical legislation in the EU has undergone a comprehensive reform as part of the European Commission's Pharmaceutical Strategy for Europe initiative launched in November 2020. Following the European Commission's publication of proposed revisions to the EU pharmaceutical framework in April 2023, the European Parliament and the Council of the European Union reached political agreement on a legislative overhaul in December 2025, and the Council formally adopted the legislative package in September 2026. The reforms remain subject to completion of the legislative process, with a multi-year transition period during which EU member states will align national laws and regulatory authorities will issue guidance. The reforms include, among other things, changes to regulatory data protection and market exclusivity periods, revised eligibility criteria for expedited and incentive-based regulatory pathways, new access and supply-related obligations, and modifications to regulatory review procedures. While the full impact of the reforms will depend on final implementing measures and their application by EU and member state authorities, the revised framework could materially affect the development, regulatory approval, exclusivity, commercialization strategy, and profitability of medicinal products in the EU, and may have a significant impact on the pharmaceutical industry and our business in the long term.

We have not obtained regulatory approval for any product candidate and it is possible that none of our product candidates in clinical programs or any other product candidates we may seek to develop in the future will ever obtain regulatory approval. Neither we nor any current or future collaborator is permitted to market any of our product candidates in the United States, the UK or the EU until we receive regulatory approval of a BLA from the FDA or of an MAA from the MHRA or European Commission, respectively. It is possible that the FDA may refuse to accept for substantive review any BLAs, or the MHRA or EMA any of our MAAs, that we submit for our product candidates or may conclude after review of our data that our application is insufficient to obtain marketing approval of our product candidates.

Prior to obtaining approval to commercialize a product candidate in the United States, the UK, the EU or elsewhere, we or our collaborators must demonstrate with substantial evidence from well-controlled clinical trials, and to the satisfaction of the FDA, MHRA, EMA or other foreign regulatory agencies, that such product candidates are safe and effective for their intended uses, or with respect to biologics in the United States, that such product candidates are safe, pure, and potent for their intended uses. Results from nonclinical studies and clinical trials can be interpreted in different ways. Even if we believe the nonclinical or clinical data for our product candidates are promising, such data may not be sufficient to support approval by the FDA, MHRA, European Commission or other regulatory authorities. The FDA, MHRA or EMA may also require us to conduct additional preclinical studies or clinical trials for our product candidates either prior to or post-approval, or it may object to elements of our clinical development program. Depending on the extent of these or any other FDA, MHRA or EMA required studies, approval of any regulatory approval applications that we submit may be delayed by several years, or may require us to expend significantly more resources than we have available.

Of the large number of potential products in development, only a small percentage successfully complete the FDA, MHRA, or other foreign regulatory approval processes and are commercialized. The lengthy approval process as well as the unpredictability of future clinical trial results may result in our failing to obtain regulatory approval to market our product candidates, which would significantly harm our business, results of operations and prospects.

Even if we obtain FDA, MHRA or European Commission approval for our product candidates in the United States, UK or EU, we may never obtain approval for or commercialize them in any other jurisdiction, which would limit our ability to realize their full market potential.

In order to market any products in any particular jurisdiction, we must establish and comply with numerous and varying regulatory requirements on a country-by-country basis regarding safety and efficacy. Approval by the FDA in the United States, the MHRA in the UK or the competent authorities in the EU does not ensure approval by regulatory authorities in other countries or jurisdictions. However, the failure to obtain approval in one jurisdiction may negatively impact our ability to obtain approval elsewhere. In addition, clinical trials conducted in one country may not be accepted by regulatory authorities in other countries, and regulatory approval in one country does not guarantee regulatory approval in any other country.

Approval processes vary among countries and can involve additional product testing and validation and additional administrative review periods. Seeking foreign regulatory approval could result in difficulties and increased costs for us and require additional preclinical studies or clinical trials which could be costly and time consuming. Regulatory requirements can vary widely from country to country and could delay or prevent the introduction of our products in those countries. We do not have any product candidates approved for sale in any jurisdiction, including in international markets, and we do not have experience in obtaining regulatory approval in international markets. If we fail to comply with regulatory requirements in international markets or to obtain and maintain required approvals, or if regulatory approvals in international markets are delayed, our target market will be reduced and our ability to realize the full market potential of any product we develop will be unrealized.

Even if we receive regulatory approval of one or more of our product candidates, we will be subject to ongoing regulatory obligations and continued regulatory review, which may result in significant additional expense, and we may be subject to penalties if we fail to comply with regulatory requirements or experience unanticipated problems with our product candidates.

Any product candidate for which we obtain marketing approval, along with the manufacturing processes, post-approval clinical data, labeling, packaging, distribution, adverse event reporting, storage, recordkeeping, export, import, advertising and promotional activities for such product, among other things, will be subject to extensive and ongoing requirements of and review by the FDA, MHRA and other regulatory authorities. These requirements include submissions of safety and other post-marketing information and reports, establishment registration and drug listing requirements, continued compliance with GMP and similar requirements relating to manufacturing, quality control, quality assurance and corresponding maintenance of records and documents, requirements regarding the distribution of samples to physicians and recordkeeping and GCP requirements for any clinical trials that we conduct post-approval.

The FDA, MHRA and other regulatory authorities closely regulate the post-approval marketing and promotion of genetic therapy medicines to ensure they are marketed only for the approved indications and in accordance with the provisions of the approved labeling. The FDA, MHRA and other regulatory authorities impose stringent restrictions on manufacturers' communications regarding off-label use and if we market our products for uses beyond their approved indications, we may be subject to enforcement action for off-label marketing. Violations of the U.S. federal Food, Drug, and Cosmetic Act, or FDCA, relating to the promotion of prescription drugs may lead to FDA enforcement actions and investigations alleging violations of federal and state healthcare fraud and abuse laws, as well as state consumer protection laws. Similar risks apply in foreign jurisdictions.

In addition, later discovery of previously unknown adverse events or other problems with our products, manufacturers or manufacturing processes, including adverse events of unanticipated severity or frequency, or with our manufacturing processes or third-party manufacturers, or failure to comply with regulatory requirements, may yield various results, including:

- restrictions on manufacturing such products;
- restrictions on the labeling or marketing of a product;
- restrictions on product distribution or use;
- requirements to conduct post-marketing studies or clinical trials;
- warning letters or holds on clinical trials;
- withdrawal of the products from the market;
- refusal to approve pending applications or supplements to approved applications that we submit;
- recall of products;
- fines, restitution or disgorgement of profits or revenues;
- suspension or withdrawal of marketing approvals;
- refusal to permit the import or export of our products;
- product seizure or detention; or
- injunctions or the imposition of civil or criminal penalties.

The FDA's and foreign regulatory authorities' policies may change and additional government regulations may be enacted that could prevent, limit or delay regulatory approval of our product candidates. We also cannot predict the likelihood, nature or extent of government regulation that may arise from future legislation or administrative action, either in the United States or in other countries. If we are slow or unable to adapt to changes in existing requirements or the adoption of new requirements or policies, or if we are not able to maintain regulatory compliance, we may be subject to enforcement action, which would adversely affect our business, prospects and ability to achieve or sustain profitability.

Interim, "topline" and preliminary data from our clinical trials that we announce or publish from time to time may change as more patient 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 may publicly disclose preliminary or topline data from our clinical trials, which is 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 related to the particular study or trial. We also make

assumptions, estimations, calculations and conclusions as part of our analyses of data, and we may not have received or had the opportunity to fully and carefully evaluate all data. As a result, the topline or preliminary results 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. Topline and preliminary data also remain subject to audit and verification procedures that may result in the final data being materially different from the topline or preliminary data we previously published. As a result, topline and preliminary data should be viewed with caution until the final data are available.

From time to time, we may also disclose interim data from our clinical trials. Interim data from these trials that we may complete are subject to the risk that one or more of the clinical outcomes may materially change as subject enrollment continues and more data become available. Adverse differences between interim data and topline, preliminary, or final data could significantly harm our business prospects. Further, disclosure of interim data by us or by our competitors could result in volatility in the price of our ordinary shares.

Further, others, including regulatory agencies, may not accept or agree with our assumptions, estimates, calculations, conclusions or analyses or may interpret or weigh the importance of data differently, which could impact the value of the particular program, the approvability or commercialization of the particular product candidate or product and our company in general. In addition, the information we choose to publicly disclose regarding a particular clinical trial is based on what is typically extensive information, and you or others may not agree with what we determine is material or otherwise appropriate information to include in our disclosure. If the interim, topline, or preliminary data that we report differ from actual results, or if others, including regulatory authorities, disagree with the conclusions reached, our ability to obtain approval for, and commercialize, our product candidates may be harmed, which could harm our business, operating results, prospects or financial condition.

We may expend our limited resources to pursue a particular product candidate or indication and fail to capitalize on product candidates or indications that may be more profitable or for which there is a greater likelihood of success.

Because we have limited financial and managerial resources, we focus on research programs and product candidates that we identify for specific indications. As a result, we may forego or delay pursuit of opportunities with other product candidates or for other indications that later prove to have greater commercial potential. Our resource allocation decisions may cause us to fail to timely capitalize on viable commercial products or profitable market opportunities. Our spending on current and future research and development programs and product candidates for specific indications may not yield any commercially viable products. If we do not accurately evaluate the commercial potential or target market for a particular product candidate, we may relinquish valuable rights to that product candidate through collaboration, licensing or other royalty arrangements in cases in which it would have been more advantageous for us to retain sole development and commercialization rights to such product candidate.

Changes in funding for, or disruptions caused by global health concerns impacting, the FDA and other government or regulatory agencies could hinder their ability to hire and retain key leadership and other personnel, or otherwise prevent new products and services from being developed, approved or commercialized in a timely manner, which could negatively impact our business.

The ability of the FDA and foreign regulatory authorities to review and approve new products can be affected by a variety of factors, including government budget and funding levels, disruptions caused by global health concerns, ability to hire and retain key personnel, including those with experience relating to novel gene therapy product candidates, acceptance of the payment of user fees, statutory, regulatory, and policy changes and other events that may otherwise affect the FDA's or foreign regulatory authorities' ability to perform routine functions. Average review times at the FDA and foreign regulatory authorities have fluctuated in recent years as a result. In addition, government funding of other government agencies that fund research and development activities is subject to the political process, which is inherently fluid and unpredictable.

Disruptions at the FDA and other government or regulatory agencies such as the EMA may also slow the time necessary for new product candidates to be reviewed and/or approved, which would adversely affect our business. For example, the U.S. government was shut down at various times during 2025 and 2026 due to Congress not having passed

appropriations bills to fund government activity, and certain regulatory agencies, such as the FDA, have had to furlough critical FDA employees and stop critical activities. Prolonged government shutdowns could significantly impact the ability of the FDA to timely review and process our regulatory submissions, which could have a material adverse effect on our business. In addition, the current presidential administration has laid off thousands of federal health workers, including at the FDA, and the leadership of CBER changed multiple times since the beginning of 2025, which may delay review times for approval of our product candidates and impact our ability to correspond with the FDA regarding the development of our programs in a timely fashion.

Risks Related to Healthcare Laws and Other Legal Compliance Matters

Enacted and future healthcare legislation may increase the difficulty and cost for us to obtain marketing approval of and commercialize our product candidates and may affect the prices we may set.

In the United States, the UK, the EU and other jurisdictions, there have been, and we expect there will continue to be, a number of legislative and regulatory changes and proposed changes to the healthcare system that could affect our future results of operations. In particular, there have been and continue to be a number of initiatives at the U.S. federal and state levels that seek to reduce healthcare costs and improve the quality of healthcare. For example, in March 2010, the Patient Protection and Affordable Care Act, as amended by the Health Care and Education Reconciliation Act, or collectively the ACA, was enacted, which substantially changed the way healthcare is financed by both governmental and private insurers. Among the provisions of the ACA, those of greatest importance to the pharmaceutical and biotechnology industries include the following:

- an annual, non-deductible fee payable by any entity that manufactures or imports certain branded prescription drugs and biologic agents (other than those designated as orphan drugs), which is apportioned among these entities according to their market share in certain government healthcare programs;
- a new methodology by which rebates owed by manufacturers under the Medicaid Drug Rebate Program are calculated for drugs that are inhaled, infused, instilled, implanted or injected;
- expansion of eligibility criteria for Medicaid programs by, among other things, allowing states to offer Medicaid coverage to certain individuals with income at or below 133% of the federal poverty level, thereby potentially increasing a manufacturer's Medicaid rebate liability;
- a licensure framework for follow on biologic products;
- a new Patient-Centered Outcomes Research Institute to oversee, identify priorities in, and conduct comparative clinical effectiveness research, along with funding for such research; and
- establishment of a Center for Medicare & Medicaid Innovation at the Centers for Medicare & Medicaid Services, or CMS, to test innovative payment and service delivery models to lower Medicare and Medicaid spending, potentially including prescription drug spending.

Since its enactment, there have been judicial, Congressional and executive branch challenges to certain aspects of the ACA. On June 17, 2021, the U.S. Supreme Court dismissed the most recent judicial challenge to the Patient Protection and Affordable Care Act, or the ACA, brought by several states without specifically ruling on the constitutionality of the ACA.

In addition, other legislative changes have been proposed and adopted in the United States since the ACA was enacted, including aggregate reductions of Medicare payments to providers, which was temporarily suspended from May 1, 2020 through March 31, 2022, and reduced payments to several types of Medicare providers. In March 2021, the American Rescue Plan Act of 2021 was signed into law, which eliminated the statutory cap on drug manufacturers' Medicaid drug rebate liability for single source and innovator multiple source drugs, beginning January 1, 2024. The rebate was previously capped at 100% of a drug's average manufacturer price.

Moreover, there has recently been heightened governmental scrutiny over the manner in which manufacturers set prices for their marketed products, which has resulted in several Congressional inquiries and proposed and enacted federal and state legislation designed to, among other things, bring more transparency to product pricing, review the relationship between pricing and manufacturer patient programs, and reform government program reimbursement methodologies for drug products. In August 2022, the Inflation Reduction Act, or IRA, was signed into law. Among other things, the IRA requires manufacturers of certain drugs to engage in price negotiations with Medicare, with prices that can be negotiated subject to a cap; imposes rebates 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 manufacturer discounting program (which began in 2025). The IRA permits the Secretary of the U.S. Department of Health and Human Services, or HHS, to implement many of these provisions through guidance, as opposed to regulation, for the initial years. CMS has published the negotiated prices for the initial ten drugs, which went into effect in 2026, and the list of the subsequent 15 drugs, which will first be effective in 2027, as well as the next set of 15 drugs that will be subject to negotiation, although the Medicare drug price negotiation program is currently subject to legal challenges. While the impact of the IRA on the pharmaceutical industry cannot yet be fully determined, it is likely to be significant. For that and other reasons, it is currently unclear how the IRA will be effectuated. These new laws or any other similar laws introduced in the future may result in additional reductions in Medicare and other healthcare funding, which could negatively affect our customers and accordingly, our financial operations.

Additionally, on July 4, 2025, the One Big Beautiful Bill Act, or OBBBA, was signed into law, which is expected to reduce Medicaid spending and enrollment by implementing work requirements for some beneficiaries, capping state-directed payments, reducing federal funding, and limiting provider taxes used to fund the program. The OBBBA also narrows access to ACA marketplace exchange enrollment and declines to extend the ACA enhanced advanced premium tax credits that expired at the end of 2025, which, among other provisions in the law, are anticipated to reduce the number of Americans with health insurance.

The Trump administration is pursuing a two-fold strategy to reduce drug costs in the U.S. President Trump has threatened to impose significant tariffs on pharmaceutical manufacturers that do not adopt pricing policies such as most favored nation pricing, which would tie the price for drugs in the U.S. to the lowest price in a group of other countries. In response, multiple manufacturers have reportedly entered into confidential pricing agreements with the federal government. The Trump administration is also pursuing traditional regulatory pathways to impose drug pricing policies, and published two proposed regulations in December 2025, referred to as Globe and Guard, which would implement mandatory payment models under which manufacturers of eligible drugs would be required to pay rebates to the federal government on a portion of the units of their drugs that are reimbursed by Medicare, with the rebate amount based on most favored nation pricing. On September 30, 2026, CMS finalized the Globe model, which applies to certain Medicare Part B drugs and is scheduled to begin on January 1, 2027. As finalized, the Globe model excludes orphan-only drugs and certain approved cell and gene therapy products, although CMS has indicated that it may reconsider the inclusion of such products through future rulemaking. The Guard proposed regulation, which would apply to certain Medicare Part D drugs, has not yet been finalized. While the impact of the Globe model and, if finalized, the Guard regulation cannot yet be determined, it is likely to be significant. Even regulatory proposals or executive actions that are ultimately deemed unlawful could negatively impact the U.S. pharmaceutical sector and our business. In addition, pharmaceutical pricing and marketing has long been the subject of considerable discussion in Congress and among policymakers, and it is possible that Congress could enact additional laws that negatively affect the pharmaceutical industry.

Payment methodologies may also be subject to changes in healthcare legislation and regulatory initiatives. For example, CMS may develop new payment and delivery models, such as bundled payment models. 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.

Individual states in the United States 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, drug price reporting and other transparency measures, and, in some cases, designed to encourage importation from other countries and bulk purchasing. Some states have enacted legislation creating so-called prescription drug affordability boards, which ultimately may

attempt to impose price limits on certain drugs in these states. Legally mandated price controls on payment amounts by third-party payors or other restrictions could harm our business, results of operations, financial condition and prospects. 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 and other healthcare programs. This could reduce the ultimate demand for our product candidates or put pressure on our product pricing.

In addition, FDA regulations and guidance may be revised or reinterpreted by the FDA in ways that may significantly affect our business and our products. Any new regulations or guidance, or revisions or reinterpretations of existing regulations or guidance, may impose additional costs or lengthen FDA review times for our product candidates. We cannot determine how changes in regulations, statutes, policies, or interpretations when and if issued, enacted or adopted, may affect our business in the future.

Such changes would likely require substantial time and impose significant costs, or could reduce the potential commercial value of our product candidates, and could materially harm our business and our financial results. In addition, delays in receipt of or failure to receive regulatory clearances or approvals for any other products would harm our business, financial condition, and results of operations.

In the UK and 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 UK or 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 UK and the EU, including the establishment and operation of health services and the pricing and reimbursement of medicines, is almost exclusively a matter for national law and policy. National governments and health service providers have different priorities and approaches to the delivery of healthcare and the pricing and reimbursement of products in that context. In general, however, the healthcare budgetary constraints in the UK and 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 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.

On December 13, 2021, Regulation No 2021/2282 on Health Technology Assessment, or HTA, amending Directive 2011/24/EU, was adopted in the EU. The Regulation entered into force in January 2022 and has been applicable since January 2025, with phased implementation based on the type of product (i.e., oncology and advanced therapy medicinal products as of 2025, orphan medicinal products as of 2028, and all other medicinal products by 2030). The Regulation intends to boost cooperation among EU member states in assessing health technologies, including new medicinal products, and provide the basis for cooperation at the EU level for joint clinical assessments in these areas. It will permit EU member states to use common HTA tools, methodologies, and procedures across the EU, working together in four main areas, including joint clinical assessment of the innovative health technologies with the highest potential impact for patients, joint scientific consultations whereby developers can seek advice from HTA authorities, identification of emerging health technologies to identify promising technologies early, and continuing voluntary cooperation in other areas. Individual EU member states will continue to be responsible for assessing non-clinical (e.g., economic, social, ethical) aspects of health technology, and making decisions on pricing and reimbursement.

In markets outside of the United States, the UK and the 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 action in the United States, the UK 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.

Our business operations and current and future relationships with investigators, healthcare professionals, consultants, third-party payors, patient organizations and customers will be subject to applicable healthcare regulatory laws, which could expose us to penalties.

Our business operations and current and future arrangements with investigators, healthcare professionals, consultants, third-party payors, patient organizations and customers, may expose us to broadly applicable fraud and abuse laws and other healthcare laws and regulations. These laws may constrain the business or financial arrangements and relationships through which we conduct our operations, including how we research, market, sell and distribute our product candidates, if approved. Such laws include:

- the U.S. federal Anti-Kickback Statute, which prohibits, among other things, persons or entities from knowingly and willfully soliciting, offering, receiving or providing any remuneration (including any kickback, bribe, or certain rebate), directly or indirectly, overtly or covertly, in cash or in kind, to induce or reward, or in return for, either the referral of an individual for, or the purchase, lease, order or recommendation of, any good, facility, item or service, for which payment may be made, in whole or in part, under U.S. federal and state healthcare programs such as Medicare and Medicaid. A person or entity does not need to have actual knowledge of the statute or specific intent to violate it in order to have committed a violation;

- the U.S. federal civil and criminal false claims and civil monetary penalties laws, including the civil False Claims Act, which, among other things, impose criminal and civil penalties, including through civil whistleblower or qui tam actions, against individuals or entities for knowingly presenting, or causing to be presented, to the U.S. federal government, claims for payment or approval that are false or fraudulent, knowingly making, using or causing to be made or used, a false record or statement material to a false or fraudulent claim, or from knowingly making a false statement to avoid, decrease or conceal an obligation to pay money to the U.S. federal government. In addition, the government may assert that a claim including items and services resulting from a violation of the U.S. federal Anti-Kickback Statute constitutes a false or fraudulent claim for purposes of the False Claims Act;

- the U.S. federal Health Insurance Portability and Accountability Act of 1996, or HIPAA, which created additional federal criminal statutes which prohibit, among other things, knowingly and willfully executing, or attempting to execute, a scheme to defraud any healthcare benefit program, or knowingly and willfully falsifying, concealing or covering up a material fact or making any materially false statement, in connection with the delivery of, or payment for, healthcare benefits, items or services. Similar to the U.S. federal Anti-Kickback Statute, a person or entity does not need to have actual knowledge of the statute or specific intent to violate it in order to have committed a violation;

- the FDCA, which prohibits, among other things, the adulteration or misbranding of drugs, biologics and medical devices;

- the U.S. Public Health Service Act, which prohibits, among other things, the introduction into interstate commerce of a biological product unless a biologics license is in effect for that product;

- federal consumer protection and unfair competition laws, which broadly regulate marketplace activities and activities that potentially harm consumers;

- the U.S. Physician Payments Sunshine Act and its implementing regulations, which requires certain manufacturers of drugs, devices, biologics and medical supplies that are reimbursable under Medicare, Medicaid, or the Children's Health Insurance Program, with specific exceptions, to report annually to the government information related to certain payments and other transfers of value to physicians (defined to include doctors, dentists, optometrists, podiatrists and chiropractors), certain non-physician practitioners (physician assistants, nurse practitioners, clinical nurse specialists, certified nurse anesthetists, anesthesiologist assistants and certified nurse midwives), and teaching hospitals, as well as ownership and investment interests held by physicians and their immediate family members;

- analogous U.S. state laws and regulations, including: state anti-kickback and false claims laws, which may apply to our business practices, including but not limited to, research, distribution, sales and marketing arrangements and claims involving healthcare items or services reimbursed by any third-party payor, including private insurers; state laws that require pharmaceutical companies to comply with the pharmaceutical industry's voluntary compliance guidelines and the relevant compliance guidance promulgated by the U.S. federal government, or otherwise restrict payments that may be made to healthcare providers and other potential referral sources; state laws and regulations that require drug manufacturers to file reports relating to pricing and marketing information, which requires tracking gifts and other remuneration and items of value provided to healthcare professionals and entities; and state and local laws that require the registration of pharmaceutical sales representatives; and

- similar healthcare laws and regulations in the UK, EU and other jurisdictions, including reporting requirements detailing interactions with and payments to healthcare providers.

Ensuring that our internal operations and future business arrangements with third parties comply with applicable healthcare laws and regulations will involve substantial costs. It is possible that governmental authorities will conclude that our business practices do not comply with current or future statutes, regulations, agency guidance or case law involving applicable fraud and abuse or other healthcare laws and regulations. If our operations are found to be in violation of any of the laws described above or any other governmental laws and regulations that may apply to us, we may be subject to significant penalties, including civil, criminal and administrative penalties, damages, fines, exclusion from government-funded healthcare programs, such as Medicare and Medicaid or similar programs in other countries or jurisdictions, integrity oversight and reporting obligations to resolve allegations of non-compliance, disgorgement, individual imprisonment, contractual damages, reputational harm, diminished profits and the curtailment or restructuring of our operations. If any of the physicians or other providers or entities with whom we expect to do business are found to not be in compliance with applicable laws, they may be subject to criminal, civil or administrative sanctions, including exclusions from government funded healthcare programs and imprisonment, which could affect our ability to operate our business. Further, defending against any such actions can be costly, time-consuming and may require significant personnel resources. Therefore, even if we are successful in defending against any such actions that may be brought against us, our business may be impaired.

We are subject to regulation and other legal obligations relating to data privacy and protection. Compliance with these requirements is complex and costly. The actual or perceived failure to comply with such obligations could materially harm our business.

The global data protection landscape is continually evolving, and we are or may become subject to numerous state, federal and foreign laws, requirements and regulations governing the collection, use, access to, confidentiality, disclosure, storage, processing, retention and security of personal information such as information that we may collect in connection with clinical trials in the U.S. and abroad.

In the U.S., HIPAA imposes privacy, security and breach reporting obligations with respect to individually identifiable health information upon "covered entities" (health plans, healthcare clearinghouses and certain healthcare providers), and their respective business associates, individuals or entities that create, receive, maintain or transmit protected health information in connection with providing a service for or on behalf of a covered entity, as well as their covered subcontractors. Most healthcare providers, including research institutions and other vendors from which we may obtain health-related information, are subject to privacy and security regulations promulgated under HIPAA. We do not believe that we are currently acting as a covered entity or business associate under HIPAA and thus are not directly subject to its requirements or penalties. However, depending on the facts and circumstances, we could face substantial criminal penalties if we knowingly receive individually identifiable health information from a HIPAA-covered healthcare provider or research institution that has not satisfied HIPAA's requirements for disclosure of individually identifiable health information.

In addition, certain state laws govern the privacy and security of health information in certain circumstances, some of which are more stringent than HIPAA and many of which differ from each other in significant ways and may not have the same effect, thus complicating compliance efforts. Failure to comply with these laws, where applicable, can result in the imposition of significant civil and/or criminal penalties and private litigation. Further, we may also be subject to other state laws governing the privacy, processing and protection of personal information. For example, the California Consumer Privacy Act, or CCPA, requires covered businesses that process the personal information of California residents to, among other things: provide certain disclosures to California residents regarding the business's collection, use, and disclosure of their personal information; receive and respond to requests from California residents to access, delete, and correct their personal information, or to opt out of certain disclosures of their personal information; and enter into specific contractual provisions with service providers that process California resident personal information on the business's behalf. Similar laws have been passed in other states, and are continuing to be proposed at the state and the federal level, reflecting a trend toward more stringent privacy legislation in the United States. HIPAA, the CCPA and other domestic privacy and data protection laws and regulations may increase our compliance costs and potential liability.

Our operations abroad may also be subject to increased scrutiny or attention from data protection authorities. For example, the General Data Protection Regulation, or GDPR, imposes stringent requirements for processing the personal data of individuals within the European Economic Area, or EEA, which consists of the 27 EU member states plus Iceland, Lichtenstein and Norway, or in the context of our activities within the EEA. Companies that must comply with the GDPR face increased compliance obligations and risk, including more robust regulatory enforcement of data protection requirements and, among other things, potential fines for noncompliance of up to €20 million or up to 4% of the total worldwide annual turnover of the relevant undertaking in the preceding financial year, whichever is higher, and other administrative penalties.

Among other requirements, the GDPR regulates transfers of personal data subject to the GDPR to third countries that have not been found to provide adequate protection to such personal data, including the U.S. Case law from the Court of Justice of the European Union states that reliance on the standard contractual clauses – a standard form of contract approved by the European Commission as an adequate personal data transfer mechanism – alone may not necessarily be sufficient in all circumstances and that transfers must be assessed on a case-by-case basis. The European Commission adopted its adequacy decision in relation to the new EU-US Data Privacy Framework ("DPF") on July 10, 2023, rendering the DPF effective as a GDPR transfer mechanism to U.S. entities self-certified under the DPF. The General Court of the European Union upheld that decision in September 2025, although adequacy decisions are subject to periodic review and may be challenged, modified, suspended or invalidated. As a result, international data transfers remain subject to legal uncertainty and heightened regulatory scrutiny. Any inability to lawfully transfer personal data across jurisdictions in which we operate could require us to implement additional safeguards, localize data or systems, or modify our operations, which could increase costs and adversely affect our business, results of operations, and financial condition.

Further, we are subject to the UK GDPR, which imposes separate but similar obligations to those under the GDPR and comparable penalties, including fines of up to £17.5 million or 4% of a noncompliant undertaking's global annual revenue for the preceding financial year, whichever is greater. On October 12, 2023, the UK Extension to the DPF came into effect (as approved by the UK Government), as a data transfer mechanism from the UK to U.S. entities self-certified under the DPF. UK privacy law has been, and continues to be, subject to scrutiny and proposed changes. This could affect the ease of data transfers between the EEA and the UK in the future. As we continue to expand into other foreign countries and jurisdictions, we may be subject to additional laws and regulations that may affect how we conduct business.

Although we work to comply with applicable laws, regulations and standards, as well as our contractual obligations and other legal obligations, relating to data privacy and security, these requirements are evolving and may be modified, interpreted and applied in an inconsistent manner from one jurisdiction and/or organization to another, and may conflict with one another or other legal obligations with which we must comply. Any failure or perceived failure by us or our employees, representatives, contractors, consultants, collaborators, or other third parties to comply with such requirements or adequately address privacy data and security concerns, even if unfounded, could result in additional costs, claims by and liability to third parties, government investigations and enforcement actions, damage to our reputation, and other adverse effects on our business, financial condition and results of operations.

We are subject to environmental, health and safety laws and regulations, and we may become exposed to liability and substantial expenses in connection with environmental compliance or remediation activities.

Our operations, including our development, testing and manufacturing activities, are subject to numerous environmental, health and safety laws and regulations. These laws and regulations govern, among other things, the controlled use, handling, release and disposal of and the maintenance of a registry for, hazardous materials and biological materials, such as chemical solvents, human cells, carcinogenic compounds, mutagenic compounds and compounds that have a toxic effect on reproduction, laboratory procedures and exposure to blood-borne pathogens. If we fail to comply with such laws and regulations, we could be subject to fines or other sanctions. Additionally, if environmental regulations are enacted that restrict our ability to use one or more of the materials or compounds necessary to manufacture our product candidates, and we are unable to find suitable alternatives or such alternatives require additional testing or will extend the manufacturing timeline, then we may be unable to manufacture our product candidates in a timely manner, or at all.

We may be subject to environmental liability inherent in our current and historical activities, including liability relating to releases of or exposure to hazardous or biological materials. Environmental, health and safety laws and regulations are becoming more stringent. We may be required to incur substantial expenses in connection with future environmental compliance or remediation activities, in which case, our production efforts or those of our third-party manufacturers may be interrupted or delayed.

Due to our international operations, we are subject to anti-corruption laws, as well as export control laws, customs laws, sanctions laws and other laws governing our operations. If we fail to comply with these laws, we could be subject to civil or criminal penalties, other remedial measures and legal expenses.

Our operations are subject to anti-corruption laws, including the UK Bribery Act 2010, or Bribery Act; the U.S. Foreign Corrupt Practices Act, or FCPA; and other anti-corruption laws that apply in countries where we do business and may do business in the future. The Bribery Act, FCPA, and these other laws generally prohibit us, our officers and our employees and intermediaries from bribing, being bribed by, or providing prohibited payments or anything else of value to government officials or other persons to obtain or retain business or gain some other business advantage. We may in the future operate in jurisdictions that pose a high risk of potential Bribery Act or FCPA violations, and we may participate in collaborations and relationships with third parties whose actions could potentially subject us to liability under the Bribery Act, FCPA, or local anti-corruption laws. In addition, we cannot predict the nature, scope, or effect of future regulatory requirements to which any of our international operations might be subject or the manner in which existing laws might be administered or interpreted.

We also are subject to other laws and regulations governing any international operations, including regulations administered by the governments of the UK and the U.S., and authorities in the EU, including applicable export control regulations, economic sanctions on countries and persons, customs requirements and currency exchange regulations, or, collectively, the Trade Control laws.

There is no assurance that we will be completely effective in ensuring our compliance with all applicable anti-corruption laws, including the Bribery Act, the FCPA, or other legal requirements, including Trade Control laws. If we are not in compliance with the Bribery Act, the FCPA, and other anti-corruption laws or Trade Control laws, we may be subject to criminal and civil penalties, disgorgement, and other sanctions and remedial measures and legal expenses. Any investigation of any potential violations of the Bribery Act, the FCPA, other anti-corruption laws, or Trade Control laws by UK, U.S., or other authorities, even if it is ultimately determined that we did not violate such laws, could be costly and time-consuming, require significant personnel resources, and harm our reputation.

We have established internal controls to detect and prevent violations of applicable anti-corruption laws and to remedy any weaknesses identified. There can be no assurance, however, that the policies and procedures will be followed at all times or effectively detect and prevent violations of the applicable laws by one or more of our employees, consultants, agents, or collaborators and, as a result, we could be subject to fines, penalties, or prosecution.

Risks Related to Commercialization

We face significant competition in an environment of rapid technological change, and there is a possibility that our competitors may achieve regulatory approval before us or develop therapies that are safer or more advanced or effective than ours, which may harm our financial condition and our ability to successfully market or commercialize any product candidates we may develop.

The development and commercialization of new gene therapy products is highly competitive. Moreover, the gene regulation and manufacturing fields are characterized by rapidly changing technologies and a strong emphasis on intellectual property. We may face competition with respect to any product candidates that we may seek to develop or commercialize in the future from major pharmaceutical companies, specialty pharmaceutical companies, and biotechnology companies worldwide. 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.

There are a number of large pharmaceutical and biotechnology companies that currently market and sell products or are pursuing the development of products for the treatment of the disease indications for which we have research programs, including inherited retinal diseases and neurodegenerative diseases. Some of these competitive products and therapies are based on scientific approaches that are similar to our approach, and others are based on entirely different approaches. Differences in the scientific approaches may create confusion or uncertainty among clinical trial investigators or patient populations, which could delay or hinder enrollment or initiation of our clinical trials.

Our platform and products focus on the development of gene therapies and gene regulation technology. In 2017, the FDA approved the first gene treatment for RPE65-associated retinal disease, Luxturna, a commercially available product developed by Spark Therapeutics, Inc., which was purchased by Roche. There are a number of other companies developing ocular gene therapy products, including Beacon Therapeutics Limited and 4D Molecular Therapeutics, Inc. There are a number of companies developing gene therapy products for neurodegenerative diseases, including Voyager Therapeutics, Inc. and Lilly. In addition to competition from other gene therapies, any products we may develop may also face competition from other types of therapies, such as small molecule, antibody, or protein therapies. Many of our current or potential competitors, either alone or with their collaboration partners, have greater financial resources and expertise in research and development, manufacturing, preclinical testing, conducting clinical trials, obtaining regulatory approvals, and marketing approved products than we do. Mergers and acquisitions in the pharmaceutical, biotechnology, and gene therapy industries may result in even more resources being concentrated among a smaller number of our competitors. These competitors also compete with us in recruiting and retaining qualified scientific, manufacturing and management personnel and establishing clinical trial sites and patient enrollment

in clinical trials, as well as in acquiring technologies complementary to, or necessary for, our programs. 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 any products that we may develop, limiting demand or the price we are able to charge, or that could render any products that we may develop obsolete or non-competitive. Our competitors also may obtain FDA, MHRA or other regulatory approval for their products more rapidly than we may obtain approval for ours, which could result in our competitors establishing a strong market position before we are able to enter the market. In addition, as a result of the expiration or successful challenge of our patent rights, we could face more litigation with respect to the validity and/or scope of patents relating to our competitors' products.

The successful commercialization of our product candidates will depend in part on the extent to which governmental authorities and health insurers establish coverage, adequate reimbursement levels and pricing policies. Failure to obtain or maintain coverage and adequate reimbursement for our product candidates, if approved, could limit our ability to market those products and decrease our ability to generate revenue.

The availability of coverage and adequacy of reimbursement by governmental healthcare programs such as Medicare and Medicaid, private health insurers and other third-party payors are essential for most patients to be able to afford medical services and pharmaceutical products such as our product candidates, assuming regulatory approval. Our ability to achieve acceptable levels of coverage and reimbursement for our products or procedures using our products by governmental authorities, private health insurers and other organizations will have an effect on our ability to successfully commercialize our product candidates. Obtaining coverage and adequate reimbursement for our products may be particularly difficult because of the higher prices often associated with drugs administered under the supervision of a physician. Separate reimbursement for the product itself or the treatment or procedure in which our product is used may not be available. A decision by a third-party payor not to cover or separately reimburse for our products or procedures using our products could reduce physician utilization of our products if approved. Assuming there is such coverage by a third-party payor, the resulting reimbursement payment rates may not be adequate or may require co-payments that patients find unacceptably high. We cannot be sure that coverage and reimbursement in the United States, the UK, the EU or elsewhere will be available for our product candidates or any product that we may develop, and any reimbursement that may become available may not be adequate or may be decreased or eliminated in the future.

Third-party payors increasingly are challenging prices charged for pharmaceutical products and services, and many third-party payors may refuse to provide coverage and reimbursement for particular drugs or biologics when an equivalent generic drug, biosimilar or a less expensive therapy is available. It is possible that a third-party payor may consider our product candidates as substitutable and only offer to reimburse patients for the less expensive product. Even if we show improved efficacy or improved convenience of administration with our product candidates, pricing of existing third-party therapeutics may limit the amount we will be able to charge for our product candidates. These payors may deny or revoke the reimbursement status of a given product or establish prices for new or existing marketed products at levels that are too low to enable us to realize an appropriate return on our investment in our product candidates. If reimbursement is not available or is available only at limited levels, we may not be able to successfully commercialize our product candidates and may not be able to obtain a satisfactory financial return on our product candidates.

There is significant uncertainty related to the insurance coverage and reimbursement of newly-approved products. In the United States, third-party payors, including private and governmental payors, such as the Medicare and Medicaid programs, play an important role in determining the extent to which new drugs and biologics will be covered. The Medicare and Medicaid programs increasingly are used as models in the United States for how private payors and other governmental payors develop their coverage and reimbursement policies for drugs and biologics. Some third-party payors may require pre-approval of coverage for new or innovative devices or drug therapies before they will reimburse healthcare providers who use such therapies. We cannot predict at this time what third-party payors will decide with respect to the coverage and reimbursement for our product candidates.

No uniform policy for coverage and reimbursement for products exists among third-party payors in the United States. Therefore, coverage and reimbursement for products can differ significantly from payor to payor. As a result, the coverage determination process is often a time-consuming and costly process that will require us to provide scientific

and clinical support for the use of our product candidates to each payor separately, with no assurance that coverage and adequate reimbursement will be applied consistently or obtained in the first instance. Furthermore, rules and regulations regarding reimbursement change frequently, in some cases on short notice.

Outside the United States, international operations are generally subject to extensive governmental price controls and other market regulations, and we believe the increasing emphasis on cost-containment initiatives in Europe and other countries have and will continue to put pressure on the pricing and usage of our product candidates. In many countries, the prices of medical products are subject to varying price control mechanisms as part of national health systems. Other countries allow companies to fix their own prices for medical products but monitor and control company profits. Additional foreign price controls or other changes in pricing regulation could restrict the amount that we are able to charge for our product candidates. Accordingly, in markets outside the United States, the reimbursement for our product candidates may be reduced compared with the United States and may be insufficient to generate commercially-reasonable revenue and profits.

Moreover, increasing efforts by governmental and third-party payors in the United States and abroad to cap or reduce healthcare costs may cause such organizations to limit both coverage and the level of reimbursement for newly approved products and, as a result, they may not cover or provide adequate payment for our product candidates. We expect to experience pricing pressures in connection with the sale of our product candidates due to the trend toward managed healthcare, the increasing influence of health maintenance organizations and additional legislative changes. The downward pressure on healthcare costs in general, particularly prescription drugs and biologics and surgical procedures and other treatments, has become intense. As a result, increasingly high barriers are being erected to the entry of new products.

Even if our product candidates receive marketing approval, they may fail to achieve market acceptance by physicians, patients, third-party payors or others in the medical community necessary for commercial success.

If our product candidates receive marketing approval, they may nonetheless fail to gain sufficient market acceptance by physicians, patients, third-party payors and others in the medical community. If they do not achieve an adequate level of acceptance, we may not generate significant product revenues or become profitable. The degree of market acceptance of our product candidates, if approved for commercial sale, will depend on a number of factors, including but not limited to:

- the efficacy and potential advantages compared to alternative treatments;
- effectiveness of sales and marketing efforts;
- the cost of treatment in relation to alternative treatments, including any similar generic treatments;
- our ability to offer our product candidates for sale at competitive prices;
- the convenience and ease of administration;
- the willingness of the target patient population to try new therapies and of physicians to prescribe these therapies;
- the strength of marketing and distribution support, and publicity concerning our products or competing products and treatments;
- the timing of market introduction of competitive products;
- the availability of third-party coverage and adequate reimbursement;
- product labeling or product insert requirements of the FDA, MHRA, EMA or other regulatory authorities, including any limitations or warnings contained in a product's approved labeling;

- the prevalence and severity of any side effects; and
- any restrictions on the use of our product together with other medications.

Because we expect sales of our product candidates, if approved, to generate substantially all of our product revenues for a substantial period, the failure of these product candidates to find market acceptance would harm our business and could require us to seek additional financing.

If we are unable to establish sales, marketing and distribution capabilities either on our own or in collaboration with third parties, we may not be successful in commercializing our product candidates or realizing the synergies in the target indications of our programs, even if they are approved.

We do not have any infrastructure for the sales, marketing or distribution of our products, and the cost of establishing and maintaining such an organization may exceed the cost-effectiveness of doing so or we may seek collaborative arrangements or external funding to commercialize our product candidates. There are significant expenses and risks involved with establishing our own sales, marketing and distribution capabilities, including our ability to hire, retain and appropriately incentivize qualified individuals, generate sufficient sales leads, provide adequate training to sales and marketing personnel, and effectively manage a geographically dispersed sales and marketing team. Any failure or delay in the development of such capabilities could delay any product launch, which would adversely impact the commercialization of our product candidates. Additionally, if any commercial launch is delayed or does not occur for any reason, we would have prematurely or unnecessarily incurred these commercialization expenses. This may be costly, and our investment would be lost if we cannot retain or reposition our sales and marketing personnel.

We may not have the resources in the foreseeable future to allocate to the sales and marketing of our product candidates in certain markets. Therefore, our future sales in these markets will largely depend on our ability to enter into and maintain collaborative relationships for such capabilities, the collaborator's strategic interest in the product and such collaborator's ability to successfully market and sell the product. For example, Lilly will be solely responsible for the commercialization of AAV-AIPL1 pursuant to the Lilly Collaboration Agreement. We also may pursue collaborative arrangements regarding the sale and marketing of bota-vec, AAV-hAQP1, AAV-GAD, our other IRD programs, our riboswitch gene regulation platform technology or other future gene therapy programs, if approved, for the United States and/or certain markets overseas; however, there can be no assurance that we will be able to establish or maintain such collaborative arrangements, or if able to do so, that they will have effective sales forces.

If we are unable to build our own sales force or negotiate or maintain a collaborative relationship for the commercialization of our product candidates, we may be forced to delay potential commercialization or reduce the scope of our sales or marketing activities. If we elect to increase our expenditures to fund commercialization activities internationally, we will need to obtain additional capital, which may not be available to us on acceptable terms, or at all. We could enter into arrangements with collaborative partners at an earlier stage than otherwise would be ideal and we may be required to relinquish rights or otherwise agree to terms unfavorable to us, any of which may have an adverse effect on our business, operating results and prospects.

Some indications targeted by our ophthalmology programs are rare, but we anticipate realizing synergies in commercializing our IRD product candidates, should they be approved. Failure to realize synergies in our sales, marketing and distribution efforts may harm our commercialization efforts.

If we or our collaborators are unable to establish or maintain adequate sales, marketing and distribution capabilities, we will not be successful in commercializing our product candidates and may not become profitable and may incur significant additional losses. We will be competing with many companies that currently have extensive and well-funded marketing and sales operations. Without an internal team or the support of a third party to perform marketing and sales functions, we may be unable to compete successfully against these more established companies.

If any of our products are commercialized outside of the United States, the UK or the EU, a variety of risks associated with international operations could adversely affect our business.

If any of our products are approved for commercialization, we have entered into, and intend to enter into, agreements with third parties to market them in certain jurisdictions outside the United States, the UK and the EU, such as under our Lilly Collaboration Agreement. We expect that we and our third-party collaborators will be subject to additional risks related to international pharmaceutical operations, including:

- different regulatory requirements for drug and biologic approvals and rules governing drug and biologic commercialization in foreign countries;
- tighter restrictions on data privacy and security and the collection and use of patient data;
- reduced or loss of protection for intellectual property rights;
- foreign reimbursement, pricing and insurance regimes;
- unexpected changes in tariffs, trade barriers and regulatory requirements;
- economic weakness, including inflation, or political instability in particular foreign economies and markets;
- foreign currency fluctuations, which could result in increased operating expenses and reduced revenues, and other obligations incident to doing business in another country;
- business interruptions resulting from geopolitical actions, including war and terrorism, or widespread health emergencies, or natural disasters including earthquakes, typhoons, floods and fires, or from economic or political instability;
- greater difficulty with enforcing our contracts;
- potential noncompliance with the FCPA, the Bribery Act and similar anti-bribery and anticorruption laws in other jurisdictions;
- production shortages resulting from any events affecting raw material supply or manufacturing capabilities abroad; and
- workforce uncertainty in countries where labor unrest is more common than in the United States and compliance with tax, employment, immigration and labor laws for employees living or traveling abroad.

We have no prior experience in these areas and we may rely on other third parties to help us establish our international commercialization operations. In addition, there are complex regulatory, tax, labor and other legal requirements imposed by individual countries in Europe with which we and our third-party collaborators will need to comply. If we are unable to successfully manage the challenges of international expansion and operations, our business and operating results could be harmed.

Any product candidates for which we intend to seek approval as biologic products may face competition sooner than anticipated.

The ACA includes a subtitle called the Biologics Price Competition and Innovation Act of 2009, or BPCIA, which created an abbreviated approval pathway for biological products that are biosimilar to or interchangeable with an FDA-licensed reference biological product. Under the BPCIA, an application for a biosimilar product may not be submitted to the FDA until four years following the date that the reference product was first licensed by the FDA. In addition, the approval of a biosimilar product may not be made effective by the FDA until 12 years from the date on

which the reference product was first licensed by the FDA. During this 12-year period of exclusivity, another company may still market a competing version of the reference product if the FDA approves a full BLA for the competing product containing the sponsor's own pre-clinical data and data from adequate and well-controlled clinical trials to demonstrate the safety, purity and potency of the other company's product.

We believe that any of our product candidates approved as a biological product under a BLA should qualify for the 12-year period of exclusivity. However, there is a risk that any of our product candidates approved as a biological product under a BLA would not qualify for the 12-year period of exclusivity or that this exclusivity could be shortened due to Congressional action or otherwise, or that the FDA will not consider our product candidates to be reference products for competing products, potentially creating the opportunity for generic competition sooner than anticipated. Jurisdictions outside the United States have established abbreviated pathways for regulatory approval of biological products that are biosimilar to earlier approved reference products. For example, the EU has had an established regulatory pathway for biosimilars since 2006. Moreover, the extent to which a biosimilar, once licensed, will be substituted for any one of our reference products in a way that is similar to traditional generic substitution for non-biological products is not yet clear, and will depend on a number of marketplace and regulatory factors that are still developing.

If competitors are able to obtain marketing approval for biosimilars referencing our products, our products may become subject to competition from such biosimilars, with the attendant competitive pressure and consequences.

Risks Related to Our Dependence on Third Parties

If our GMP manufacturing facilities are unable to supply our product candidates for all of our current preclinical, clinical and potential commercial needs, including any anticipated third party supply obligations, we will be forced to seek out third-party manufacturers. We currently contract with third parties for the manufacture of plasmid used in producing product candidates. Relying on third parties increases the risk that we will not have sufficient quantities of such materials, product candidates, or any medicines that we may develop and commercialize, or that such supply will not be available to us at an acceptable cost, which could delay, prevent, or impair our development or commercialization efforts.

We produce our product candidates in our GMP viral vector manufacturing facility in London, UK, and our second, large scale GMP viral vector manufacturing facility and our first GMP DNA production facility in Shannon, Ireland. However, if our current facilities are damaged, suffer any form of delay or regulatory challenges, we experience slowdowns or problems with our facilities or we are unable to scale our internal manufacturing capabilities to meet demand for our product candidates, we will need to contract with third-party manufacturers to produce our product candidates. We rely on third-party manufacturers for the manufacture of plasmid used in the production of certain product candidates. We do not have a long-term supply agreement with any of the third-party manufacturers, and we purchase our required supply on a purchase order basis.

We and our third-party manufacturers may also encounter difficulties or delays in manufacturing of our product candidates or the plasmid used in the production of our product candidates. Geopolitical actions, natural disaster or a widespread health emergency could impact our supply chain. To the extent that we or our third-party manufacturers are located in geographies affected by these matters, it may result in the temporary closing of manufacturing facilities and may increase the costs associated with manufacturing our product candidates.

We may be unable to establish any agreements with third-party manufacturers or to do so on acceptable terms. Even if we are able to establish agreements with third-party manufacturers, reliance on third-party manufacturers entails additional risks, including:

- the possible breach of the manufacturing agreement by the third party, including failure to provide appropriate quantities in a timely manner;
- the possible termination or nonrenewal of the agreement by the third party at a time that is costly or inconvenient for us; and

- reliance on the third party for regulatory compliance, quality assurance, safety, and pharmacovigilance and related reporting.

We and our third-party manufacturers may not be able to comply with GMP regulations or similar regulatory requirements that might be required by the FDA, MHRA or EMA. Our failure, or the failure of our third-party manufacturers, to comply with applicable regulations could result in sanctions being imposed on us, including fines, injunctions, civil penalties, delays, suspension or withdrawal of approvals, license revocations, seizures or recalls of product candidates or medicines, operating restrictions, and criminal prosecutions, any of which could adversely affect supplies of our candidates and harm our business, financial condition, results of operations, and prospects.

Any therapies that we may develop may compete with other product candidates and products for access to manufacturing facilities. There are a limited number of manufacturers that operate under GMP or similar regulations and that might be capable of manufacturing for us. Any performance failure on the part of our existing or future manufacturers could delay clinical development or marketing approval.

Our current and anticipated future dependence upon others for the manufacture of any product candidates we may develop or any components required for the manufacture of our product candidates may adversely affect our future profit margins and our ability to commercialize any product candidates that receive marketing approval on a timely and competitive basis.

We have in the past, and may in the future, collaborate with third parties for the development, manufacture and commercialization of our product candidates. We may not succeed in establishing and maintaining collaborative relationships, which may significantly limit our ability to develop and commercialize our product candidates successfully, if at all.

We have entered into collaboration agreements with third parties for the development and commercialization of our product candidates, including the Lilly Collaboration Agreement for the development and commercialization of AAV-AIPL1 and two other preclinical product candidates which are intended to treat other inherited retinal dystrophies. In addition, in October 2023 we provided Sanofi and its affiliates with a right of first negotiation for use of our riboswitch gene regulation technology for certain Immunology and Inflammation (I&I), including modulation of IL-4 and IL-13, and Central Nervous System (CNS) targets, as well as for GLP-1 and other gut peptides for metabolic disease, and for our Phase 2 xerostomia program, under the Investment Agreement. We also completed the initial closing of our strategic collaboration with Hologen in April 2026 as described above. We may seek additional collaborative relationships in the future. Failure to obtain a collaborative relationship for our product candidates may significantly impair their commercial potential. We also may need to enter into collaborative relationships to provide funding to support our other research and development programs. The process of establishing and maintaining collaborative relationships is difficult, time-consuming and involves significant uncertainty, such as:

- a collaboration partner may shift its priorities and resources away from our product candidates due to a change in business strategies, or a merger, acquisition, sale or downsizing;
- a collaboration partner may seek to renegotiate or terminate their relationships with us due to unsatisfactory clinical results, manufacturing issues, a change in business strategy, a change of control or other reasons;
- a collaboration partner may cease development in therapeutic areas which are the subject of our strategic collaboration;
- a collaboration partner may not devote sufficient capital or resources towards our product candidates;
- a collaboration partner may change the success criteria for a product candidate thereby delaying or ceasing development of such candidate;

- a significant delay in initiation of certain development activities by a collaboration partner will also delay payment of milestones tied to such activities, thereby impacting our ability to fund our own activities;
- a collaboration partner could develop a product that competes, either directly or indirectly, with our product candidate;
- a collaboration partner with commercialization obligations may not commit sufficient financial or human resources to the marketing, distribution or sale of a product;
- a collaboration partner with manufacturing responsibilities may encounter regulatory, resource or quality issues and be unable to meet demand requirements;
- a collaboration partner may terminate a strategic alliance;
- a dispute may arise between us and a partner concerning the research, development or commercialization of a product candidate resulting in a delay in milestones, royalty payments or termination of an alliance and possibly resulting in costly litigation or arbitration which may divert management attention and resources; and
- a partner may use our products or technology in such a way as to make us subject to litigation with a third party.

If any collaborator fails to fulfill its responsibilities in a timely manner, or at all, our research, clinical development, manufacturing or commercialization efforts related to that collaboration could be delayed or terminated, or it may be necessary for us to assume responsibility for expenses or activities that would otherwise have been the responsibility of our collaborator. If we are unable to establish and maintain collaborative relationships on acceptable terms or to successfully transition terminated collaborative agreements, we may have to delay or discontinue further development of one or more of our product candidates, undertake development and commercialization activities at our own expense or find alternative sources of capital.

We have relied, and we expect to continue to rely, on third parties to conduct, supervise and monitor our preclinical studies and clinical trials, and if these third parties perform in an unsatisfactory manner, our business could be harmed.

We expect to rely on CROs, clinical trial sites, and other vendors to ensure our preclinical studies and clinical trials are conducted properly and on time. We may also engage third parties such as clinical data management organizations, medical institutions and clinical investigators to conduct or assist in our clinical trials or other preclinical and clinical research and development work. While we will have agreements governing their activities, we will have limited influence over their actual performance. We will control only certain aspects of our third-party service providers' activities. Nevertheless, we will be responsible for ensuring that each of our preclinical studies and clinical trials is conducted in accordance with applicable protocol, legal, quality, regulatory and scientific standards, including among other things, GCP requirements for clinical trials and good laboratory practice requirements for certain preclinical studies. Our reliance on these third parties does not relieve us of our regulatory responsibilities. For example, we are conducting the Phase 2 AQUAx2 clinical trial of AAV-hAQP1 for the treatment of patients with radiation-induced xerostomia at multiple clinical trial sites in North America and the United Kingdom. If any locations terminate the clinical trial, we may be required to find another party to conduct any new trials. We may be unable to find a new party to conduct new trials of our product candidates or obtain clinical supply of our product candidates or AAV vectors for such trials. If we elect to internalize some or all activities related to the conduct of our preclinical studies or clinical trials that are currently performed by our third-party service providers, or if we are required to do so due to a service provider's termination of our relationship, then we may be required to source additional technology and personnel in order to perform the relevant activities. We may be unsuccessful in our efforts to internalize some or all relevant activities, either on the desired timeline or at all.

Our third-party service providers are not our employees, and therefore we may be unable to directly monitor whether or not they devote sufficient time, attention, expertise and resources to our clinical and nonclinical programs. These third-party service providers may also have relationships with other commercial entities, including our competitors, for whom they may also be conducting clinical trials or other drug development activities that could harm our competitive position. If our third-party service providers do not successfully carry out their contractual duties or obligations or fail to meet expected deadlines, or if the quality or accuracy of the preclinical or clinical data they obtain is compromised due to the failure to adhere to our clinical protocols or regulatory requirements, or for any other reasons, our preclinical studies or clinical trials may be extended, delayed or terminated, and we may not be able to obtain regulatory approval for, or successfully commercialize our product candidates. As a result, our financial results and the commercial prospects for our product candidates could be harmed, our costs could increase, and our ability to generate revenues could be delayed.

If our relationship with any CROs terminate, we may not be able to enter into arrangements with alternative CROs or do so on commercially reasonable terms. Switching or adding additional CROs involves substantial cost and requires management time and focus. In addition, there is a natural transition period when a new CRO commences work. As a result, delays occur, which can materially impact our ability to meet our desired clinical development timelines. Though we intend to carefully manage our relationships with our CROs, 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 an adverse impact on our business, financial condition and prospects.

Risks Related to Intellectual Property

We depend on proprietary technology licensed from others. If we lose our existing licenses or are unable to acquire or license additional proprietary rights from third parties, we may not be able to continue developing our product candidates.

We currently in-license certain intellectual property from research institutions, universities and other third parties. We may also enter into additional agreements, including license agreements, with other parties in the future that impose diligence, development and commercialization timelines, milestone payments, royalties, insurance and other obligations on us. If we fail to comply with our obligations to any of our current or future collaborators, our counterparties may have the right to terminate these agreements, in which event we might not be able to develop, manufacture or market any product candidate that is covered by these agreements, which could adversely affect the value of the product candidate being developed under any such agreement. Termination of these agreements or reduction or elimination of our rights under these agreements may result in our having to negotiate new or reinstated agreements with less favorable terms, or cause us to lose our rights under these agreements, including our rights to important intellectual property or technology.

We may rely on other third parties from whom we license proprietary technology to file and prosecute patent applications and maintain patents and otherwise protect the intellectual property we license from them. We may have limited control over these activities or any other intellectual property that may be related to our in-licensed intellectual property. For example, we cannot be certain that such activities by these licensors will be conducted in compliance with applicable laws and regulations or will result in valid and enforceable patents and other intellectual property rights. We may have limited control over the manner in which our licensors initiate an infringement proceeding against a third-party infringer of the intellectual property rights, or defend certain of the intellectual property that may be licensed to us. It is possible that the licensors' infringement proceedings or defense activities may be less vigorous than if we conduct them ourselves. The licensing and acquisition of third-party intellectual property rights is a competitive practice, and companies that may be more established, or have greater resources than we do, may also be pursuing strategies to license or acquire third-party intellectual property rights that we may consider necessary or attractive in order to commercialize our product candidates. More established companies may have a competitive advantage over us due to their larger size and cash resources or greater clinical development and commercialization capabilities. There can be no assurance that we will be able to successfully complete such negotiations and ultimately acquire the rights to the intellectual property surrounding the additional product candidates that we may seek to acquire.

If we are unable to obtain and maintain patent protection for our technology and product candidates or if the scope of the patent protection obtained is not sufficiently broad, we may not be able to compete effectively in our markets.

We rely upon a combination of patents, trade secret protection and confidentiality agreements to protect the intellectual property related to our proprietary technologies, product candidate development programs and product candidates. Our success depends in part on our ability to secure and maintain patent protection in the United States and other countries with respect to our current product candidates and any future product candidates we may develop. We seek to protect our proprietary position by filing or collaborating with our licensors to file patent applications in the United States and abroad related to our proprietary technologies, development programs and product candidates. The patent prosecution process is expensive and time-consuming, and we may not be able to file and prosecute all necessary or desirable patent applications at a reasonable cost or in a timely manner. Moreover, the issuance, scope, validity, enforceability and commercial value of our patent rights are uncertain.

It is also possible that we might fail to identify patentable aspects of our research and development output before it is too late to obtain patent protection. We may not have the right to control the preparation, filing, and prosecution of patent applications, or to maintain the rights to patents licensed to third parties. Therefore, these patents and patent applications may not be prosecuted and enforced in a manner consistent with the best interests of our business. The patent applications that we own or in-license may fail to result in issued patents with claims that cover our proprietary products and technology, including current product candidates, any future product candidates we may develop, and our gene regulation technology in the United States or in other countries, in whole or in part. Alternately, our existing patents and any future patents we obtain may not be sufficiently broad to prevent others from using our technology or from developing competing products and technologies. There is no assurance that all potentially relevant prior art relating to our patents and patent applications has been found, which can prevent a patent from issuing from a pending patent application or later invalidate or narrow the scope of an issued patent. For example, publications of discoveries in the scientific literature often lag behind the actual discoveries, and patent applications in the United States and other jurisdictions are typically not published until 18 months after filing or, in some cases, not at all. Therefore, we cannot know with certainty whether we were the first to make the inventions claimed in our patents or pending patent applications, or that we were the first to file for patent protection of such inventions. In addition, obtaining and maintaining our patent protection depends on compliance with various procedural, document submission, fee payment and other requirements imposed by governmental patent agencies, and our patent protection could be reduced or eliminated for non-compliance with these requirements. Even if patents do successfully issue and even if such patents cover our current product candidates, any future product candidates we may develop and our gene regulation technology, third parties may challenge their validity, enforceability or scope thereof, which may result in such patents being narrowed, invalidated, or held unenforceable. Any successful challenge to these patents or any other patents owned by or licensed to us could deprive us of rights necessary for the successful commercialization of any of our product candidates or gene regulation technology. Our competitors may be able to circumvent our patents by developing similar or alternative product candidates in a non-infringing manner. Further, if we encounter delays in regulatory approvals, the period of time during which we could market a product candidate and our gene regulation technology under patent protection could be reduced.

If the patent applications we hold or have in-licensed with respect to our development programs and product candidates fail to issue, if their validity, breadth or strength of protection is threatened, or if they fail to provide meaningful exclusivity for any of our current or future product candidates or technology, it could dissuade companies from collaborating with us to develop product candidates, encourage competitors to develop competing products or technologies and threaten our ability to commercialize future product candidates. Any such outcome could harm our business.

The patent position of biotechnology and pharmaceutical companies is uncertain, involves complex legal and factual questions, and is characterized by the existence of large numbers of patents and frequent litigation based on allegations of patent or other intellectual property infringement or violation. In addition, the laws of jurisdictions outside the United States may not protect our rights to the same extent as the laws of the United States. Changes in either the patent laws or interpretation of the patent laws in the United States and other countries may diminish the value of our patents or narrow the scope of our patent protection.

The issuance of a patent is not conclusive as to its inventorship, scope, validity or enforceability, and our owned and licensed patents may be challenged in the courts or patent offices in the United States and abroad. Such challenges may result in loss of exclusivity or freedom to operate or in patent claims being narrowed, invalidated or held unenforceable, in whole or in part, which could limit our ability to stop others from using or commercializing similar or identical technology and products, or limit the duration of the patent protection of our technology and products. Thus, even if our patent applications issue as patents, they may not issue in a form that will provide us with meaningful protection, prevent competitors from competing with us or otherwise provide us with any competitive advantage. Moreover, patents have a limited lifespan. In the United States, the natural expiration of a patent is generally 20 years after it is filed. Various extensions may be available; however, the life of a patent, and the protection it affords, is limited. Without patent protection for our current or future product candidates, we may be open to competition from generic versions of such products. 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.

Third parties may assert claims against us alleging infringement of their patents and proprietary rights, or we may need to become involved in lawsuits to defend or enforce our patents, either of which could result in substantial costs or loss of productivity, delay or prevent the development and commercialization of our product candidates, prohibit our use of proprietary technology or sale of products or put our patents and other proprietary rights at risk.

Our commercial success depends, in part, upon our ability to develop, manufacture, market and sell our product candidates without alleged or actual infringement, misappropriation or other violation of the patents and proprietary rights of third parties. However, our research, development and commercialization activities may be subject to claims that we infringe or otherwise violate patents or other intellectual property rights owned or controlled by third parties. Litigation relating to infringement or misappropriation of patent and other intellectual property rights in the pharmaceutical and biotechnology industries is common, including patent infringement lawsuits, interferences, oppositions and *inter partes* reviews, and reexamination proceedings before the U.S. Patent and Trademark Office, or USPTO, and corresponding foreign patent offices. In addition, many companies in intellectual property-dependent industries, including the biotechnology and pharmaceutical industries, have employed intellectual property litigation as a means to gain an advantage over their competitors. Numerous U.S., EU and foreign issued patents and pending patent applications, which are owned by third parties, exist in the fields in which we are developing product candidates, and as the biotechnology and pharmaceutical industries expand and more patents are issued, the risk increases that our product candidates may be subject to claims of infringement of the intellectual property rights of third parties. Some claimants may have substantially greater resources than we do and may be able to sustain the costs of complex intellectual property litigation to a greater degree and for longer periods of time than we could. In addition, patent holding companies that focus solely on extracting royalties and settlements by enforcing patent rights may target us.

We may be subject to third-party claims including infringement, interference or derivation proceedings, post-grant review and *inter partes* review before the USPTO or similar adversarial proceedings or litigation in other jurisdictions. Even if such claims are without merit, a court of competent jurisdiction could hold that these third-party patents are valid, enforceable and infringed, and the holders of any such patents may be able to block our ability to commercialize the applicable product candidate unless we obtained a license under the applicable patents, or until such patents expire or are finally determined to be invalid or unenforceable. In addition, third parties may obtain patents in the future and claim that use of our technologies infringes upon these patents, and the holders of any such patents may be able to prohibit our use of those compositions, formulations, methods of treatment, prevention or use or other technologies, effectively blocking our ability to develop and commercialize the applicable product candidate until such patent expires or is finally determined to be invalid or unenforceable or unless we obtained a license.

In addition, defending such claims would cause us to incur substantial expenses and, if we are not successful in defending such claims, it could cause us to pay substantial damages if we are found to be infringing a third party's patent rights. These damages potentially include increased damages (possibly treble damages) and attorneys' fees if we are found to have infringed such rights willfully. Further, if a patent infringement suit is brought against us or our third-party service providers, our development, manufacturing or sales activities relating to the product or product candidate that is the subject of the suit may be delayed or terminated. As a result of patent infringement claims, or in order to avoid

potential infringement claims, we may choose to seek, or be required to seek, a license from the third party, which may require payment of substantial royalties or fees, or require us to grant a cross-license under our intellectual property rights. These licenses may not be available on reasonable terms or at all. Even if a license can be obtained on reasonable terms, the rights may be nonexclusive, which would give our competitors access to the same intellectual property rights. If we are unable to enter into a license on acceptable terms, we could be prevented from commercializing one or more of our product candidates, or forced to modify such product candidates, or to cease some aspect of our business operations, which could harm our business significantly. We might also be forced to redesign or modify our product candidates so that we no longer infringe the third-party intellectual property rights, which may result in significant cost or delay to us, or which redesign or modification could be impossible or technically infeasible. Even if we were ultimately to prevail, any of these events could require us to divert substantial financial and management resources that we would otherwise be able to devote to our business.

Competitors may infringe our patents or other intellectual property. If we or one of our licensors were to initiate legal proceedings against a third party to enforce a patent covering one of our product candidates, the defendant could counterclaim that our patent is invalid or unenforceable. If a defendant were to prevail on a legal assertion of invalidity or unenforceability, we would lose at least part, and perhaps all, of the patent protection on our product candidates.

Even if resolved in our favor, litigation or other legal proceedings relating to intellectual property claims may cause us to incur significant expenses and could distract our technical and management personnel from their normal responsibilities. In addition, 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. Such litigation or proceedings could substantially increase our operating losses and reduce our resources available for development activities. We may not have sufficient financial or other resources to adequately conduct such litigation or proceedings. Some of our competitors may be able to sustain the costs of such litigation or proceedings more effectively than we can because of their substantially greater financial resources. Uncertainties resulting from the initiation and continuation of patent litigation or other proceedings could have an adverse effect on our ability to compete in the marketplace.

We may not identify relevant third-party patents or may incorrectly interpret the relevance, scope or expiration of a third-party patent, which might adversely affect our ability to develop, manufacture and market our product candidates.

We cannot guarantee that any of our or our licensors' patent searches or analyses, including but not limited to the identification of relevant patents, analysis of the scope of relevant patent claims or determination of the expiration of relevant patents, are complete or thorough, nor can we be certain that we have identified each and every third-party patent and pending application in the United States, the UK, the EU and elsewhere that is relevant to or necessary for the commercialization of our product candidates in any jurisdiction. For example, in the United States, applications filed before November 29, 2000 and certain applications filed after that date that will not be filed outside the United States remain confidential until patents issue. Patent applications in the United States, the UK, the EU and elsewhere are published approximately 18 months after the earliest filing for which priority is claimed, with such earliest filing date being commonly referred to as the priority date. Therefore, patent applications covering our product candidates could be filed by others without our knowledge. Additionally, pending patent applications that have been published can, subject to certain limitations, be later amended in a manner that could cover our product candidates or the use of our product candidates. After issuance, the scope of patent claims remains subject to construction as determined by an interpretation of the law, the written disclosure in a patent and the patent's prosecution history. Our interpretation of the relevance or the scope of a patent or a pending application may be incorrect, which may negatively impact our ability to market our product candidates. We may incorrectly determine that our product candidates are not covered by a third-party patent or may incorrectly predict whether a third party's pending application will issue with claims of relevant scope. Our determination of the expiration date of any patent in the United States, the UK, the EU or elsewhere that we consider relevant may be incorrect, which may negatively impact our ability to develop and market our product candidates. Our failure to identify and correctly interpret relevant patents may negatively impact our ability to develop and market our product candidates.

If we fail to correctly identify or interpret relevant patents, we may be subject to infringement claims. We cannot guarantee that we will be able to successfully settle or otherwise resolve such infringement claims. If we fail in any such dispute, in addition to being forced to pay monetary damages, we may be temporarily or permanently prohibited from commercializing our product candidates. We might, if possible, also be forced to redesign our product candidates in a manner that no longer infringes third-party intellectual property rights. Any of these events, even if we were ultimately to prevail, could require us to divert substantial financial and management resources that we would otherwise be able to devote to our business.

Changes in patent laws or patent jurisprudence could diminish the value of patents in general, thereby impairing our ability to protect our product candidates.

Obtaining and enforcing patents in the biotechnology and genetic medicine industries involve both technological complexity and legal complexity. In addition, the Leahy-Smith America Invents Act, or the AIA, which was passed in September 2011, resulted in significant changes to the U.S. patent system.

An important change introduced by the AIA is that, as of March 16, 2013, the United States transitioned from a "first-to-invent" to a "first-to-file" system for deciding which party should be granted a patent when two or more patent applications are filed by different parties claiming the same invention. Under a "first-to-file" system, assuming the other requirements for patentability are met, the first inventor to file a patent application generally will be entitled to a patent on the invention regardless of whether another inventor had made the invention earlier. A third party that files a patent application in the USPTO after that date but before us could therefore be awarded a patent covering an invention of ours even if we made the invention before it was made by the third party. This will require us to be cognizant of the time from invention to filing of a patent application and diligent in filing patent applications, but circumstances could prevent us from promptly filing patent applications on our inventions.

In addition, a third party may attempt to use the USPTO procedures to invalidate our patent claims that would not have been invalidated if first challenged by the third party as a defendant in a district court action because of a lower evidentiary standard in USPTO proceedings compared to the evidentiary standard in U.S. federal courts necessary to invalidate a patent claim. An adverse determination in any such proceeding could reduce the scope of, or invalidate, our owned or in-licensed patent rights, allow third parties to commercialize our technology or products and compete directly with us, without payment to us, or result in our inability to manufacture or commercialize products without infringing third-party patent rights.

Additionally, the U.S. Supreme Court has ruled on several patent cases in recent years either narrowing the scope of patent protection available in certain circumstances or weakening the rights of patent owners in certain situations, and there are other open questions under patent law that courts have yet to decisively address. In addition to increasing uncertainty with regard to our ability to obtain patents in the future, this combination of events has created uncertainty with respect to the value of patents, once obtained. Depending on decisions by Congress, the federal courts and the USPTO, the laws and regulations governing patents could change in unpredictable ways and could weaken our ability to obtain new patents or to enforce our existing patents and patents that we might obtain in the future. In addition, the European patent system is relatively stringent in the type of amendments that are allowed during prosecution, but, the complexity and uncertainty of European patent laws has also increased in recent years. Complying with these laws and regulations could limit our ability to obtain new patents that may be important for our business.

We enjoy only limited geographical protection with respect to certain patents and we may not be able to protect our intellectual property rights throughout the world.

Filing, prosecuting and defending patents covering our product candidates in all countries throughout the world would be prohibitively expensive, and our intellectual property rights in some countries outside the United States can be less extensive than those in the United States. In-licensing patents covering our product candidates in all countries throughout the world may similarly be prohibitively expensive, if such opportunities are available at all. And in-licensing or filing, prosecuting and defending patents even in only those jurisdictions in which we develop or commercialize our product candidates may be prohibitively expensive or impractical. Competitors may use our and our licensors' technologies in jurisdictions where we have not obtained patent protection or licensed patents to develop their

own products and, further, may export otherwise infringing products to territories where we and our licensors have patent protection, but enforcement is not as strong as that in the United States, the UK or the EU. These products may compete with our product candidates, and our or our licensors' patents or other intellectual property rights may not be effective or sufficient to prevent them from competing.

The laws of some jurisdictions do not protect intellectual property rights to the same extent as the laws or regulations in the United States, the UK and the EU, and many companies have encountered significant difficulties in protecting and defending proprietary rights in such jurisdictions. Moreover, the legal systems of certain countries, particularly certain developing countries, do not favor the enforcement of patents, trade secrets or other forms of intellectual property, which could make it difficult for us to prevent competitors in some jurisdictions from marketing competing products in violation of our proprietary rights generally. Proceedings to enforce our patent rights in foreign jurisdictions, whether or not successful, are likely to result in substantial costs and divert our efforts and attention from other aspects of our business, and additionally could put at risk our or our licensors' patents of being invalidated or interpreted narrowly, could increase the risk of our or our licensors' patent applications not issuing, or could provoke third parties to assert claims against us. We may not prevail in any lawsuits that we initiate, while damages or other remedies may be awarded to the adverse party, which may be commercially significant. If we prevail, damages or other remedies awarded to us, if any, may not be commercially meaningful. Accordingly, our efforts, or the efforts of our licensors or collaborators, to enforce intellectual property rights around the world may be inadequate to obtain a significant commercial advantage from the intellectual property that we develop or license.

Patent terms may be inadequate to protect our competitive position on our product candidates for an adequate amount of time.

The term of any individual patent depends on applicable law in the country where the patent is granted. In the United States, provided all maintenance fees are timely paid, a patent generally has a term of 20 years from its application filing date or earliest claimed non-provisional filing date. Extensions may be available under certain circumstances, but the life of a patent and, correspondingly, the protection it affords is limited. Even if we or our licensors obtain patents covering our product candidates, when the terms of all patents covering a product expire, our business may become subject to competition from competitive medications, including generic medications. Given the amount of time required for the development, testing and regulatory review and approval of new product candidates, patents protecting such candidates may 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.

If we do not obtain patent term extension in the United States under the Hatch-Waxman Act and in foreign countries under similar legislation, thereby potentially extending the term of marketing exclusivity for our product candidates, our business may be harmed.

In the United States, a patent that covers an FDA-approved drug or biologic may be eligible for a term extension designed to restore the period of the patent term that is lost during the premarket regulatory review process conducted by the FDA. Depending upon the timing, duration and conditions of FDA marketing approval of our product candidates, one or more of our U.S. patents may be eligible for limited patent term extension under the Drug Price Competition and Patent Term Restoration Act of 1984, or the Hatch-Waxman Act, which permits a patent term extension of up to five years for a patent covering an approved product as compensation for effective patent term lost during product development and the FDA regulatory review process. In the UK and the EU, our product candidates may be eligible for term extensions based on similar legislation. In each of these jurisdictions, however, we may not receive an extension if we fail to apply within applicable deadlines, fail to apply prior to expiration of relevant patents or otherwise fail to satisfy applicable requirements. Even if we are granted such extension, the duration of such extension may be less than our request. If we are unable to obtain a patent term extension, or if the term of any such extension is less than our request, the period during which we can enforce our patent rights for that product will be essentially shortened and our competitors may obtain approval to market competing products sooner. The resulting reduction in revenue from applicable products could be substantial.

Our proprietary rights may not adequately protect our technologies and product candidates, and 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 our competitive advantage. The following examples are illustrative:

- others may be able to make products that are the same as or similar to our product candidates but that are not covered by the claims of the patents that we own or have exclusively licensed;
- others, including inventors or developers of our owned or in-licensed patented technologies who may become involved with competitors, may independently develop similar technologies that function as alternatives or replacements for any of our technologies without infringing our intellectual property rights;
- we or our licensors or our other collaboration partners might not have been the first to conceive and reduce to practice the inventions covered by the patents or patent applications that we own, license or will own or license;
- we or our licensors or our other collaboration partners might not have been the first to file patent applications covering certain of the patents or patent applications that we or they own or have obtained a license, or will own or will have obtained a license;
- we or our licensors may fail to meet obligations to the U.S. government with respect to in-licensed patents and patent applications funded by U.S. government grants, leading to the loss of patent rights;
- issued patents that we own or exclusively license may not provide us with any competitive advantage, or may be held invalid or unenforceable, as a result of legal challenges by our competitors; and
- our competitors might conduct research and development activities in countries where we do not have patent rights, or in countries where research and development safe harbor laws exist, and then use the information learned from such activities to develop competitive products for sale in our major commercial markets.

Our reliance on third parties may require us to share our trade secrets, which increases the possibility that our trade secrets will be misappropriated or disclosed, and confidentiality agreements with employees and third parties may not adequately prevent disclosure of trade secrets and protect other proprietary information.

We consider proprietary trade secrets, confidential know-how and unpatented know-how to be important to our business. We may rely on trade secrets and confidential know-how to protect our technology, especially where patent protection is believed by us to be of limited value. However, trade secrets and confidential know-how are difficult to protect, and we have limited control over the protection of trade secrets and confidential know-how used by our licensors, collaborators and suppliers. Because we have relied in the past on third parties to manufacture our product candidates, because we may continue to do so in the future, and because we expect to collaborate with third parties on the development of our current product candidates and any future product candidates we develop, we may, at times, share trade secrets with them. 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. Under such circumstances, trade secrets and confidential know-how can be difficult to maintain as confidential.

To protect this type of information against disclosure or appropriation by competitors, our policy is to require our employees, consultants, contractors and advisors to enter into confidentiality agreements and, if applicable, material transfer agreements, consulting agreements or other similar agreements with us 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, including our trade secrets. However, current or former employees, consultants, contractors and advisers may unintentionally or willfully disclose our confidential information to competitors, and confidentiality agreements may not provide an adequate remedy in the event of unauthorized disclosure of confidential information. We may also be subject to claims that our employees, consultants or independent contractors have wrongfully used or disclosed confidential information of their former employers or other 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 competitive 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 an adverse effect on our business and results of operations. Enforcing a claim that a third party obtained illegally and is using trade secrets and/or confidential know-how is expensive, time consuming and unpredictable, and the enforceability of confidentiality agreements may vary from jurisdiction to jurisdiction. Courts outside the United States are sometimes less willing to protect proprietary information, technology and know-how.

If our trademarks and trade names are not adequately protected, then we may not be able to build name recognition in our markets of interest and our business may be adversely affected.

If our trademarks and trade names are not adequately protected, then we may not be able to build name recognition in our markets of interest and our business may be adversely affected. Our trademark MeiraGTx is the subject of registrations and/or pending applications in the EU, UK and United States. We also own, and may seek to register, trademarks and trade names that we may use in connection with our product candidates and any products that we may commercialize in the future. We may not be able to protect our rights to these trademarks and trade names, which we need to build name recognition among potential partners or customers in our markets of interest. At times, competitors may adopt trade names or trademarks similar to ours, thereby impeding our ability to build brand identity and possibly leading to market confusion. In addition, there could be potential trade name or trademark infringement claims brought by owners of other registered trademarks or trademarks that incorporate variations of our registered or unregistered trademarks or trade names. We may not be able to obtain or maintain registrations for our trademarks in all jurisdictions in which we may seek to commercialize our product candidates, and third parties may oppose or otherwise challenge our applications or registrations. Over the long term, if we are unable to successfully register our trademarks and trade names and establish name recognition based on our trademarks and trade names, then we may not be able to compete effectively and our business may be adversely affected. Our efforts to enforce or protect our proprietary rights related to trademarks, trade secrets, domain names, copyrights or other intellectual property may be ineffective and could result in substantial costs and diversion of resources and could adversely impact our financial condition or results of operations.

We may need to license or acquire additional intellectual property from third parties, and such intellectual property may not be available or may not be available on commercially reasonable terms.

The growth of our business may depend in part on our ability to acquire or in-license additional proprietary rights. For example, our programs may involve product candidates or equipment that may require the use of additional proprietary rights held by third parties. Our product candidates may also require specific formulations to work effectively and efficiently. These formulations may be covered by intellectual property rights held by others. We may develop products containing our compositions and pre-existing pharmaceutical compositions. These pharmaceutical products may be covered by intellectual property rights held by others. We may be required by the FDA, MHRA, EMA or other foreign regulatory authorities to provide a companion diagnostic test or tests with our product candidates. These diagnostic test or tests may be covered by intellectual property rights held by others. We may be unable to acquire or in-license any relevant third-party intellectual property rights that we identify as necessary or important to our business operations. We may fail to obtain any of these licenses at a reasonable cost or on reasonable terms, if at all, which would harm our business. We may need to cease use of the compositions or methods covered by such third-party intellectual property rights, and may need to seek to develop alternative approaches that do not infringe on such intellectual property

rights which may entail additional costs and development delays, even if we were able to develop such alternatives, which may not be feasible. Even if we are able to obtain a license under such intellectual property rights, any such license may be non-exclusive, which may allow our competitors access to the same technologies licensed to us.

Risks Related to Employee Matters and Managing Our Organization

We may need to increase or decrease the size of our organization, and we may experience difficulties in managing those organizational changes, which could disrupt our operations.

As of September 30, 2026, we had 386 employees. If we seek to expand our organization, we may have difficulty identifying, hiring and integrating new personnel. Future growth would impose significant additional responsibilities on our management, including the need to identify, recruit, maintain, motivate and integrate additional employees, consultants and contractors. Also, our management may need to divert a disproportionate amount of its attention away from our day-to-day activities and devote a substantial amount of time to managing these growth activities. We may not be able to effectively manage the expansion of our operations, which may result in weaknesses in our infrastructure, give rise to operational mistakes, loss of business opportunities or strategic opportunities related to our assets, loss of employees and reduced productivity among remaining employees. Our growth could require significant capital expenditures and may divert financial resources from other projects, such as the development of product candidates. If our management is unable to effectively manage our growth, our expenses may increase more than expected, our ability to generate and/or grow revenues could be reduced, and we may not be able to implement our business strategy. Our future financial performance and our ability to commercialize our product candidates and compete effectively will depend, in part, on our ability to effectively manage any future growth. Our growth could require significant capital expenditures and may divert financial resources from other projects, such as the development of additional product candidates. If our management is unable to effectively manage our growth, our expenses may increase more than expected, our potential ability to generate revenue could be reduced and we may not be able to implement our business strategy. Many of the biotechnology companies that we compete against for qualified personnel and consultants have greater financial and other resources, different risk profiles and a longer history in the industry than we do. If we are unable to continue to attract and retain high-quality personnel and consultants, the rate and success at which we can discover and develop product candidates and operate our business will be limited. Alternatively, if we seek to decrease the number of employees in our organization in the future in response to adverse business events, it may lead to additional unanticipated attrition. If our future staffing is inadequate because of additional unanticipated attrition or because we failed to retain the staffing level required to accomplish our business objectives, we may be delayed or unable to continue the development of our product candidates, which could impede our ability to generate revenues and achieve or maintain profitability.

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

Our industry has experienced a high rate of turnover of management personnel in recent years. We are highly dependent on the development, regulatory, commercialization and business development expertise of Alexandria Forbes, Ph.D., our President and Chief Executive Officer, Rich Giroux, our Chief Operating Officer and Chief Financial Officer and the other executive officers and principal members of our management, scientific and clinical teams. Although we have formal employment agreements with certain of our executive officers, these agreements do not prevent them from terminating their employment with us at any time and, for certain of our executive officers, entitle them to receive severance payments in connection with their voluntary resignation of employment.

If we lose one or more of our executive officers or key employees, our ability to implement our business strategy successfully could be seriously harmed. Furthermore, replacing executive officers and key employees may be difficult and may take an extended period of time because of the limited number of individuals in our industry with the breadth of skills and experience required to develop, gain regulatory approval of and commercialize product candidates successfully. Competition to hire from this limited pool is intense, and we may be unable to hire, train, retain or motivate these additional key personnel on acceptable terms given the competition among numerous pharmaceutical and biotechnology companies for similar personnel. In addition, we rely on consultants and advisors, including scientific and clinical advisors, to assist us in formulating our research and development and commercialization strategy. Our

consultants and advisors may be engaged by entities other than us and may have commitments under consulting or advisory contracts with other entities that may limit their availability to us. If we are unable to continue to attract and retain high quality personnel, our ability to develop and commercialize product candidates will be limited.

Potential product liability lawsuits against us could cause us to incur substantial liabilities and limit commercialization of any products that we may develop.

The use of our product candidates in clinical trials and the sale of any products for which we obtain marketing approval exposes us to the risk of product liability claims. Product liability claims might be brought against us by consumers, healthcare providers, pharmaceutical companies or others selling or otherwise coming into contact with our products. On occasion, large judgments have been awarded in class action lawsuits based on products that had unanticipated adverse effects. If we cannot successfully defend against product liability claims, we could incur substantial liability and costs. In addition, regardless of merit or eventual outcome, product liability claims may result in:

- impairment of our business reputation and significant negative media attention;
- withdrawal of participants from our clinical trials;
- significant time, costs and diversion of management resources to defend the related litigation;
- substantial monetary awards to patients or other claimants;
- inability to commercialize our product candidates;
- product recalls, withdrawals or labeling, marketing or promotional restrictions;
- decreased demand for our product candidates, if approved for commercial sale; and
- loss of revenue.

Our insurance policies are expensive and protect us only from some business risks, which leaves us exposed to significant uninsured liabilities.

We do not carry insurance for all categories of risk that our business may encounter. Some of the policies we currently maintain include general liability, clinical product and clinical trial liability, employment practices liability, property, transit, auto, workers' compensation, umbrella, cyber and directors' and officers' insurance. Any additional product liability insurance coverage we acquire in the future may not be sufficient to reimburse us for any expenses or losses we may suffer. Moreover, insurance coverage is becoming increasingly expensive and restrictive, and in the future we may not be able to maintain insurance coverage at a reasonable cost or in sufficient amounts to protect us against losses due to liability. If we obtain marketing approval for our product candidates or manufacture commercial products for third parties, we intend to acquire insurance coverage to include, as necessary, the sale, manufacture and supply of commercial products; however, we may be unable to obtain product liability insurance on commercially reasonable terms or in adequate amounts. A successful product liability claim or series of claims brought against us could cause our share price to decline and, if judgments exceed our insurance coverage, could adversely affect our results of operations and business, including preventing or limiting the commercialization of any product candidates we develop. We do not carry specific biological or hazardous waste insurance coverage, and our property, casualty and general liability insurance policies specifically exclude coverage for damages and fines arising from biological or hazardous waste exposure or contamination. Accordingly, in the event of contamination or injury, we could be held liable for damages or be penalized with fines in an amount exceeding our resources, and our clinical trials or regulatory approvals could be suspended.

Operating as a public company may make it more difficult and more expensive for us to obtain directors' and officers' liability insurance, and we may be required to accept reduced policy limits and coverage or incur substantially higher costs to obtain the same or similar coverage. As a result, it may be more difficult for us to attract and retain qualified people to serve on our board of directors, our board committees or as executive officers. If we are unable to maintain existing insurance with adequate levels of coverage, any significant uninsured liability may require us to pay substantial amounts, which would adversely affect our cash position and results of operations.

Our employees and independent contractors, including consultants, vendors, and any third parties we may engage in connection with development and commercialization may engage in misconduct or other improper activities, including noncompliance with regulatory standards and requirements, which could harm our business.

Misconduct by our employees and independent contractors, including consultants, vendors, and any third parties we may engage in connection with development and commercialization, could include intentional, reckless or negligent conduct or unauthorized activities that violate: (i) applicable laws and regulations of the FDA, MHRA, EMA and other regulatory or governmental authorities, including those laws that require the reporting of true, complete and accurate information to such authorities; (ii) manufacturing standards; (iii) data privacy and security, fraud and abuse and other healthcare laws and regulations; or (iv) laws that require the reporting of true, complete and accurate financial information and data. Specifically, sales, marketing and business arrangements in the healthcare industry are subject to extensive laws and regulations intended to prevent fraud, misconduct, kickbacks, self-dealing and other abusive practices. These laws and regulations may restrict or prohibit a wide range of pricing, discounting, marketing and promotion, sales commission, customer incentive programs and other business arrangements. Activities subject to these laws could also involve the improper use or misrepresentation of information obtained in the course of clinical trials, creation of fraudulent data in preclinical studies or clinical trials or illegal misappropriation of drug product, which could result in regulatory sanctions and cause serious harm to our reputation. It is not always possible to identify and deter misconduct by employees and other third parties, and the precautions we take to detect and prevent this activity may not be effective in controlling unknown or unmanaged risks or losses or in protecting us from governmental investigations or other actions or lawsuits stemming from a failure to comply with such laws or regulations. Additionally, we are subject to the risk that a person or government could allege such fraud or other misconduct, even if none occurred. If any such actions are instituted against us, and we are not successful in defending ourselves or asserting our rights, those actions could have a significant impact on our business and results of operations, including the imposition of significant civil, criminal and administrative penalties, damages, monetary fines, disgorgements, possible exclusion from participation in Medicare, Medicaid, other U.S. federal healthcare programs or healthcare programs in other jurisdictions, integrity oversight and reporting obligations to resolve allegations of non-compliance, individual imprisonment, other sanctions, contractual damages, reputational harm, diminished profits and future earnings, and curtailment of our operations.

Our business and operations may suffer in the event of system failures and our systems and those of our business partners and service providers may be vulnerable to cybersecurity risks.

Our information technology, or IT, systems, including manufacturing systems, as well as those of our business partners and service providers, are vulnerable to damage from computer viruses, unauthorized access, hardware and software failures, natural disasters, terrorism, war and telecommunication and electrical failures. If such an event were to occur, it could result in a material disruption of our product candidate development programs or manufacturing operations. For example, the loss of preclinical study or clinical trial data from completed, ongoing or planned trials could result in delays in our regulatory approval efforts and significantly increase our costs to recover or reproduce the data. A significant interruption to our manufacturing operations could delay the completion of clinical trials and increase the costs of those trials, or impede our ability to meet any anticipated third party supply obligations. To the extent that any disruption or security breach were to result in a loss of or damage to our data or applications, or inappropriate disclosure of personal, confidential or proprietary information, we could incur liabilities and the further development of our product candidates could be delayed.

In the ordinary course of our business, we, our business partners and our service providers collect, process and store sensitive data, including intellectual property, clinical trial data, proprietary business information, personal data and personally identifiable information of our clinical trial subjects and employees. The secure processing, maintenance and transmission of this information is critical to our operations. Increased cybersecurity threats pose a risk to this

information, in addition to our and our business partners' and service providers' systems and networks. Cybersecurity threats including state-sponsored attacks, ransomware, phishing, insider threats, supply chain compromises, and vulnerabilities in cloud-based services, are increasing in frequency, sophistication, persistence and severity. Attackers, ranging from criminal organizations to nation-state actors, may target our systems for financial, competitive, or political motives. We may also face increased cybersecurity risks due to our reliance on internet technology and by allowing some of our employees to work remotely, which may create additional opportunities for cybercriminals to exploit vulnerabilities. Furthermore, because the techniques used to obtain unauthorized access to, or to sabotage, systems change frequently and often are not recognized until launched against a target, we may be unable to anticipate these techniques or implement adequate preventative measures. We may also experience security breaches that may remain undetected for an extended period. Even if identified, we may be unable to adequately investigate or remediate incidents or breaches due to attackers increasingly using tools and techniques that are designed to circumvent controls, to avoid detection, and to remove or obfuscate forensic evidence.

Despite our security measures, our IT and infrastructure may be vulnerable to cyber-attacks by hackers or internal bad actors, or breached due to employee error, a technical vulnerability, malfeasance or other disruptions that could have a negative impact, including loss or destruction of data, including confidential or critical business information. In addition, there can be no assurance that our cybersecurity risk management program and processes, including our policies, controls or procedures, will be fully effective in protecting our systems and information. Although, to our knowledge, we have not experienced any such material security breach to date, we may experience cybersecurity incidents such as malware infections, ransomware, phishing attempts, thefts of personal, confidential, proprietary or other critical business information and other attempts at compromising our IT that are typical for a company of our size in our market. Any security breach could compromise our networks and the information stored there could be accessed, publicly disclosed, lost or stolen. Any such access, disclosure or other loss of information could result in legal claims or proceedings, liability under laws that protect the privacy of personal information, significant regulatory penalties, and such an event could disrupt our operations, damage our reputation, result in significant expenses in implementing future security measures and cause a loss of confidence in us and our ability to conduct clinical trials, which could adversely affect our reputation and financial results, and delay clinical development of our product candidates.

The use or anticipated use of new and evolving technologies, such as artificial intelligence ("AI"), by us or third parties may increase or create new operational risks.

We have in the past and will in the future integrate new and evolving technologies, such as AI, into our business. AI technologies offer numerous potential benefits, such as creating or increasing operational efficiencies, and we expect an increase in the use of AI and generative AI by us, third parties on our behalf, and other market actors, including our competitors. For example, as part of our strategic collaboration, Hologen will contribute its proprietary multi-modal generative AI foundation models (LMMs) to the joint venture to develop the Clinical Programs and the Delivery Device, as well as deploy their AI capabilities to further accelerate the optimization of our proprietary manufacturing capabilities. However, the deployment of such technologies also poses certain risks, including that the algorithms may be flawed, misused or otherwise function in an unexpected manner; data sets may be insufficient, of poor quality, or contain biased information; and inappropriate or controversial data practices by data scientists, engineers, and end-users could impair results. In addition, use of personal data in generative AI technologies is subject to various privacy laws and other privacy obligations in certain jurisdictions. Governments have passed and may pass additional laws regulating generative AI. The introduction of AI technologies into our operations may potentially result in new or enhanced governmental or regulatory scrutiny, litigation, confidentiality or security risks or other complications. The regulatory landscape governing AI technologies is evolving rapidly, and changes in laws, regulations or enforcement practices may potentially impose new compliance requirements, restrict certain AI applications or increase regulatory obligations.

If the analyses that AI-based applications assist in producing are or are perceived to be deficient, inaccurate or biased, we could be subjected to competitive harm, potential legal liability and brand or reputational harm. Our competitors may also adopt AI or generative AI more quickly or more effectively than we do, which could cause competitive harm. Furthermore, use of AI-based software may lead to the unauthorized disclosure of confidential information which may impact our ability to realize the benefits of our intellectual property.

The rapid evolution of AI will require the application of significant resources to design, develop, test, oversee and maintain our products and services to help ensure that AI is implemented in accordance with applicable law and regulation and in a socially responsible manner and to minimize any real or perceived unintended harmful impacts. Our vendors or partners may in turn incorporate AI tools into their own offerings, and the providers of these AI tools may not meet existing or rapidly evolving regulatory or industry standards, including with respect to privacy and data security. Further, bad actors around the world use increasingly sophisticated methods, including the use of AI, to engage in illegal activities involving the theft and misuse of personal information, confidential information and intellectual property. Any of these effects could damage our reputation, result in the loss of valuable property and information, cause us to breach applicable laws and regulations, and adversely impact our business.

Risks Related to Our Ordinary Shares

The market price of our ordinary shares may be volatile and fluctuate substantially, which could result in substantial losses for purchasers of our ordinary shares.

Our share price is likely to be volatile. The stock market in general and the market for smaller biopharmaceutical companies in particular have experienced extreme volatility that has often been unrelated to the operating performance of particular companies. As a result of this volatility, you may not be able to sell your ordinary shares at or above your purchase price. The market price for our ordinary shares may be influenced by many factors, including:

- the success of competitive products or technologies;
- actual or expected changes in our growth rate relative to our competitors;
- results of clinical trials of our product candidates or those of our competitors;
- developments related to our existing or any future collaborations;
- regulatory or legal developments in the United States and other countries;
- development of new product candidates that may address our markets and make our product candidates less attractive;
- changes in physician, hospital or healthcare provider practices that may make our product candidates less useful;
- announcements by us, our partners or our competitors of significant acquisitions, strategic partnerships, joint ventures, collaborations or capital commitments;
- the impact of any potential strategic transactions related to our assets;
- developments or disputes concerning patent applications, issued patents or other proprietary rights;
- the recruitment or departure of key personnel;
- the level of expenses related to any of our product candidates or clinical development programs;
- failure to meet or exceed financial estimates and projections of the investment community or that we provide to the public;
- the results of our efforts to discover, develop, acquire or in-license additional product candidates or products;

- actual or expected changes in estimates as to financial results, development timelines, recommendations by securities analysts or shifting investor perceptions;
- 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;
- general economic, industry and market conditions;
- changes in accounting principles; and
- the other factors described in this "Item 1A. Risk Factors" section and elsewhere in this Form 10-Q.

In addition, the stock market in general, and Nasdaq and biopharmaceutical companies in particular, have experienced extreme price and volume fluctuations that have often been unrelated or disproportionate to the operating performance of these companies. In the past, when the market price of a security has been volatile, holders of that security have sometimes instituted securities class action litigation against the issuer. This risk is especially relevant for us because biopharmaceutical companies have experienced significant stock price volatility in recent years. If any of the holders of our ordinary shares were to bring such a lawsuit against us, we could incur substantial costs defending the lawsuit and the attention of our senior management would be diverted from the operation of our business. Any adverse determination in litigation could also subject us to significant liabilities. Broad market and industry factors may negatively affect the market price of our ordinary shares, as well as general economic, political and market conditions such as recessions, interest rate changes or international currency fluctuations, regardless of our actual operating performance. Further, a decline in the financial markets and related factors beyond our control may cause the price of our ordinary shares to decline rapidly and unexpectedly. If the market price of our ordinary shares does not exceed your purchase price, you may not realize any return on your investment in us and may lose some or all of your investment.

We may raise additional capital pursuant to a shelf registration statement, including through an "at-the-market" offering program, or through additional public or private placements, any of which could substantially dilute the investment of our stockholders.

Sales of a substantial number of our ordinary shares in the public market could dilute your ownership interest. Pursuant to an "at-the-market" sales agreement we entered into with BofA Securities, Inc., or BofA, in December 2023, we may sell from time to time, ordinary shares having an aggregate offering price of up to $100.0 million through BofA, acting as our agent. During the nine-month period ended September 30, 2026, we raised gross proceeds of $60.8 million through the sale of 5,574,968 ordinary shares pursuant to an "at-the-market" equity offering program. We may establish additional "at-the-market" equity offering programs or otherwise sell additional ordinary shares pursuant to existing or future shelf registration statements. Whether we choose to affect future sales under an "at-the-market" equity offering program will depend on a number of factors, including, among others, market conditions and the trading price of our ordinary shares relative to other sources of capital. The issuance from time to time of ordinary shares through an "at-the-market" equity offering program or in any other equity offering, or the perception that such sales may occur, could have the effect of depressing the market price of our ordinary shares.

Our executive officers, directors and principal shareholders, if they choose to act together, have the ability to significantly influence all matters submitted to shareholders for approval.

As of September 30, 2026, our executive officers, directors and shareholders who owned more than 5% of our outstanding ordinary shares and their respective affiliates, in the aggregate, hold ordinary shares representing approximately 43.36% of our outstanding ordinary shares. In addition, an affiliate of Perceptive Advisors LLC, our largest shareholder that employs a director serving on our board, holds warrants to purchase an aggregate of 700,000 of our ordinary shares.

As a result, if these shareholders choose to act together, they would be able to significantly influence all matters submitted to our shareholders for approval, as well as our management and affairs. For example, these persons, if they choose to act together, would significantly influence the election of directors, the composition of our management and approval of any merger, consolidation, sale of all or substantially all of our assets or other business combination that other shareholders may desire. Any of these actions could adversely affect the market price of our ordinary shares.

We are a "smaller reporting company," and the reduced disclosure requirements applicable to smaller reporting companies may make our ordinary shares less attractive to investors.

We are a smaller reporting company, and we will remain a smaller reporting company until the fiscal year following the determination that our voting and non-voting ordinary shares held by non-affiliates is more than $250 million measured on the last business day of our second fiscal quarter, or our annual revenues are more than $100 million during the most recently completed fiscal year and our voting and non-voting ordinary shares held by non-affiliates is more than $700 million measured on the last business day of our second fiscal quarter. Smaller reporting companies are able to provide simplified executive compensation disclosure, are exempt from the auditor attestation requirements of Section 404, and have certain other reduced disclosure obligations, including, among other things, not being required to provide selected financial data, supplemental financial information or risk factors.

We may choose to take advantage of some, but not all, of the available exemptions for smaller reporting companies. We cannot predict whether investors will find our ordinary shares less attractive if we rely on these exemptions. If some investors find our ordinary shares less attractive as a result, there may be a less active trading market for our ordinary shares and our share price may be more volatile.

Anti-takeover provisions in our organizational documents and Cayman Islands law may discourage or prevent a change of control, even if an acquisition would be beneficial to our shareholders, which could depress the price of our ordinary shares and prevent attempts by our shareholders to replace or remove our current management.

Our memorandum and articles of association contain provisions that may discourage unsolicited takeover proposals that shareholders may consider to be in their best interests. Our board of directors is divided into three classes with staggered, three-year terms. Our board of directors has the ability to designate the terms of and issue preferred shares without shareholder approval. We are also subject to certain provisions under Cayman Islands law that could delay or prevent a change of control. Together these provisions may make more difficult the removal of management and may discourage transactions that otherwise could involve payment of a premium over prevailing market prices for our ordinary shares.

There may be difficulties in enforcing foreign judgments against our management or us.

Certain of our directors and management reside outside the United States. A significant portion of our assets and such persons' assets are located outside the United States. As a result, it may be difficult or impossible for investors to effect service of process upon us within the United States or other jurisdictions, including judgments predicated upon the civil liability provisions of the federal securities laws of the United States.

In particular, investors should be aware that there is uncertainty as to whether the courts of the Cayman Islands or any other applicable jurisdictions would recognize and enforce judgments of U.S. courts obtained against us or our directors or management predicated upon the civil liability provisions of the securities laws of the United States or any state in the United States or entertain original actions brought in the Cayman Islands or any other applicable jurisdiction's courts against us or our directors or officers predicated upon the securities laws of the United States or any state in the United States.

The rights of our shareholders differ from the rights typically offered to shareholders of a U.S. corporation.

Our corporate affairs and the rights of holders of ordinary shares are governed by Cayman Islands law, including the provisions of the Cayman Islands Companies Act (as amended), or the Companies Act, the common law of the Cayman Islands and by our memorandum and articles of association. We are also subject to the federal securities

laws of the United States. The rights of shareholders to take action against the directors, actions by minority shareholders and the fiduciary responsibilities of our directors to us under Cayman Islands law are to a large extent governed by the common law of the Cayman Islands. The common law of the Cayman Islands is derived in part from comparatively limited judicial precedent in the Cayman Islands as well as from English common law, the decisions of whose courts are of persuasive authority, but are not binding on a court in the Cayman Islands. The rights of our shareholders and the fiduciary responsibilities of our directors under Cayman Islands law are different from what they would be under statutes or judicial precedent in some jurisdictions in the United States. In particular, the Cayman Islands has a different body of securities laws as compared to the United States, and certain states, such as Delaware, may have more fully developed and judicially interpreted bodies of corporate law. In addition, Cayman Islands companies may not have standing to initiate a shareholders derivative action in a Federal court of the United States.

As a result of all of the above, public shareholders may have more difficulty in protecting their interests in the face of actions taken by management, members of the board of directors or controlling shareholders than they would as public shareholders of a United States company.

We expect to be treated as resident in the UK for tax purposes, but may be treated as a dual resident company for UK tax purposes.

Our board of directors conducts our affairs so that the central management and control of the company is exercised in the UK. As a result, we expect to be treated as resident in the UK for UK tax purposes. Accordingly, we expect to be subject to UK taxation on our income and gains, except where an exemption applies.

However, we may be treated as a dual resident company for UK tax purposes. As a result, our right to claim certain reliefs from UK tax may be restricted, and changes in law or practice in the UK could result in the imposition of further restrictions on our right to claim UK tax reliefs.

We may be classified as a passive foreign investment company, or PFIC, for U.S. federal income tax purposes, which could result in adverse U.S. federal income tax consequences to U.S. investors in our ordinary shares.

Based on the current and anticipated value of our assets, including goodwill, and the current and anticipated composition of our income, assets and operations, we do not believe we were a PFIC for the taxable year ended on December 31, 2025, and do not expect to be a PFIC for the current taxable year. However, the application of the PFIC rules is subject to uncertainty in several respects, and we cannot assure you that the U.S. Internal Revenue Service, or the IRS, will not take a contrary position. Furthermore, a separate determination must be made after the close of each taxable year as to whether we are a PFIC for that year. Accordingly, we cannot assure you that we were not a PFIC for our taxable year ended on December 31, 2025 or that we will not be a PFIC for our current taxable year or any future taxable year. A non-U.S. company will be considered a PFIC for any taxable year if (i) at least 75% of its gross income is passive income (including interest income), or (ii) at least 50% of the value of its assets (based on an average of the quarterly values of the assets during a taxable year) is attributable to assets that produce or are held for the production of passive income. The value of our assets generally is determined by reference to the market price of our ordinary shares, which may fluctuate considerably. In addition, the composition of our income and assets is affected by how, and how quickly, we spend any cash we raise. If we were to be classified as a PFIC for any taxable year during which a U.S. holder holds our ordinary shares, certain materially adverse U.S. federal income tax consequences could apply to such U.S. holder.

If a United States person is treated as owning at least 10% of our ordinary shares, such holder may be subject to adverse U.S. federal income tax consequences.

If a U.S. holder of our ordinary shares is treated as owning (directly, indirectly or constructively) at least 10% of the value or voting power of our ordinary shares, such U.S. holder may be treated as a "United States shareholder" with respect to each "controlled foreign corporation" in our group (if any). If our group includes one or more U.S. subsidiaries, certain of our non-U.S. subsidiaries could be treated as controlled foreign corporations (regardless of whether we are treated as a controlled foreign corporation). A United States shareholder of a controlled foreign corporation may be required to report annually and include in its U.S. taxable income its pro rata share of "Subpart F

income," "global intangible low-taxed income" and investments in U.S. property by controlled foreign corporations, regardless of whether we make any distributions. An individual that is a United States shareholder with respect to a controlled foreign corporation generally would not be allowed certain tax deductions or foreign tax credits that would be allowed to a United States shareholder that is a U.S. corporation. Failure to comply with these reporting obligations may subject you to significant monetary penalties and may prevent the statute of limitations from starting with respect to your U.S. federal income tax return for the year for which reporting was due. We cannot provide any assurances that we will assist investors in determining whether any of our non-U.S. subsidiaries is treated as a controlled foreign corporation or whether such investor is treated as a United States shareholder with respect to any of such controlled foreign corporations. Further, we cannot provide any assurances that we will furnish to any United States shareholders information that may be necessary to comply with the aforementioned reporting and tax payment obligations. U.S. holders of our ordinary shares should consult their tax advisors regarding the potential application of these rules to their investment in our ordinary shares.

Changes in tax laws or challenges to our tax position could adversely affect our results of operations and financial condition.

We are subject to complex tax laws that are subject to change or differing interpretations, including on a retroactive basis. Any such changes in tax laws, regulations and treaties, or the interpretation thereof, tax policy initiatives and reforms under consideration and the practices of tax authorities in jurisdictions in which we operate could adversely affect our tax position, including our effective tax rate or tax payments.

We have significant U.S. federal and state net operating losses, or NOLs, and UK and Ireland carryforward tax losses which we may not be able to realize or which may be restricted under applicable law. We also benefit from certain tax incentive regimes, such as research and development tax credits. Any adverse change to these regimes, the application thereof or challenges to the tax position we have adopted under these rules could adversely affect our results of operations and financial condition.

As of December 31, 2025, we had federal and state NOL carryforwards in the United States of $163.5 million and $23.4 million, respectively, and cumulative carryforward tax losses in the UK of $196.0 million, which we expect to be available to reduce future taxable income subject to any relevant restrictions (including those in the U.S. and UK that limit the percentage of taxable income that can be reduced by NOLs and carried forward losses). As of December 31, 2025, cumulative carryforward tax losses in Ireland were $118.1 million. U.S. federal NOLs generated after December 31, 2017 are not subject to expiration but such NOLs may only offset 80% of taxable income for taxable years beginning after December 31, 2020. U.S. federal NOLs generated prior to December 31, 2017 may only be carried forward for 20 years. As of December 31, 2025, we also had orphan drug and research and development credits in the U.S. in the amount of $27.2 million, both of which may be carried forward for 20 years and are expected to begin expiring in 2035 if not utilized. We also had research and development credits in the UK of $5.9 million, which may be carried forward indefinitely until utilized. The UK and Ireland carryforward tax losses will continue indefinitely, subject to relevant restrictions, under current jurisdictional tax law.

The NOLs and carryforward tax losses are subject to review and possible adjustment by the applicable tax authorities. Additionally, carryforward tax losses, and research and development tax credits, may become subject to limitations in the event of certain cumulative changes in the ownership interest of significant shareholders, as determined under Sections 382 of the United States Internal Revenue Code, as well as the Corporation Tax Act 2010 Part 14 under the UK tax rules and the Taxes Consolidation Act 1997 (TCA 1997) under the Ireland tax rules. This could limit the amount of NOLs or carryforward tax losses that we can utilize annually to offset future taxable income or tax liabilities. We have conducted a review of changes in the ownership interest of significant shareholders and determined that as of December 2024, there were no limitations in the UK. However, for U.S. purposes, we determined that a change of ownership occurred in April 2016 and again in June 2018, but there was not a limit for utilizing these losses to offset the 2022 income. We have performed a 382 analysis through December 2024 and no additional change of ownership has occurred. Subsequent ownership changes and changes to the U.S. federal or state or UK tax rules in respect of the utilization of NOLs and carryforward tax losses may further affect the limitation in future years.

General Risk Factors

We may engage in acquisitions that could disrupt our business, cause dilution to our shareholders or reduce our financial resources.

We have, and may in the future, enter into transactions to acquire other businesses, products or technologies. For example, in July 2025, we acquired through a French insolvency proceeding certain assets, operations and employees of Smart Immune, a French clinical-stage biotechnology company that developed ProTcell, a T-cell progenitor-based cell therapy platform that harnesses the patient's own thymus to rapidly re-arm the immune system against a wide range of potential conditions, including cancer and autoimmune conditions. We may not be able to successfully integrate these acquired assets, operations and personnel into our existing business in an effective, timely and nondisruptive manner. If we do identify suitable candidates, we may not be able to make such acquisitions on favorable terms, or at all. Any acquisitions we make may not strengthen our competitive position, and these transactions may be viewed negatively by customers or investors. We may decide to incur debt in connection with an acquisition or issue our ordinary shares or other equity securities to the shareholders of the acquired company, which would reduce the percentage ownership of our existing shareholders. We could incur losses resulting from undiscovered liabilities of the acquired business that are not covered by the indemnification we may obtain from the seller. Acquisitions may also divert management attention from day-to-day responsibilities, increase our expenses and reduce our cash available for operations and other uses. We cannot predict the number, timing or size of future acquisitions or the effect that any such transactions might have on our operating results.

Exchange rate fluctuations may adversely affect our results of operations and financial condition.

Owing to the international scope of our operations, fluctuations in exchange rates may adversely affect us, particularly between the U.S. dollar on the one hand, and the pound sterling and euro on the other hand. As a result, our business and the market price of our securities may be affected by such fluctuations, which may have a significant impact on our results of operations and cash flows from period to period. Currently, we do not have any exchange rate hedging arrangements in place.

Our management team has broad discretion as to the use of the net proceeds from public and private equity or debt financings and the investment of these proceeds may not yield a favorable return. We may invest the proceeds in ways with which our shareholders disagree.

We have broad discretion in the application of any net proceeds we have received in the past or may receive in the future pursuant to existing or future equity and debt financings, including under our "at-the-market" equity offering program. Shareholders may not agree with our decisions, and our use of the proceeds and our existing cash and cash equivalents may not improve our results of operation or enhance the value of our ordinary shares. Our ability to apply certain proceeds may be restricted. For example, in April 2026, we conducted an equity financing by selling 11.1 million ordinary shares at a price of $9.00 per share for gross proceeds of approximately $100.0 million. Our failure to apply any such funds effectively could have a material adverse effect on our business, delay the development of our product candidates and cause the market price of our ordinary shares to decline. In addition, until the net proceeds are used, they may be placed in investments that do not produce significant income or that may lose value. Additionally, our existing cash and cash equivalents are subject to general credit, liquidity, market and interest rate risks, which have been and may, in the future, be exacerbated by a U.S. and/or global financial crises. We may realize losses in the fair value of certain of our investments or a complete loss of these investments if the credit markets tighten, which would have an adverse effect on our results of operations, liquidity and financial condition.

We incur substantial costs as a result of operating as a public company, and our management is required to devote substantial time to new and existing compliance initiatives and corporate governance practices.

As a public company, we incur and will continue to incur significant legal, accounting and other expense
s. The Sarbanes-Oxley Act of 2002, the Dodd-Frank Wall Street Reform and Consumer Protection Act, The Nasdaq Global Select listing requirements and other applicable securities rules and regulations impose various requirements on public companies, including establishment and maintenance of effective disclosure and financial controls and corporate governance practices. Our management and other personnel need to devote a substantial amount

of time to these compliance initiatives. Moreover, these rules and regulations increase our legal and financial compliance costs.

Pursuant to Section 404 of the Sarbanes-Oxley Act of 2002, or Section 404, we are required to furnish a report by our management on our internal control over financial reporting. However, while we are a non-accelerated filer, we will not be required to include an attestation report on internal control over financial reporting issued by our independent registered public accounting firm. To achieve compliance with Section 404, we engage in a process to document and evaluate our internal control over financial reporting, which is both costly and challenging. In this regard, we will need to continue to dedicate internal resources, potentially engage outside consultants, adopt a detailed work plan to assess and document the adequacy of internal control over financial reporting, continue steps to improve control processes as appropriate, validate through testing whether such controls are functioning as documented, and implement a continuous reporting and improvement process for internal control over financial reporting. Despite our efforts, there is a risk that we, or our independent registered public accounting firm, if we no longer qualify as a non-accelerated filer, will not be able to conclude that our internal control over financial reporting is effective as required by Section 404. In addition, any testing by us conducted in connection with Section 404, or any subsequent testing by our independent registered public accounting firm, may reveal deficiencies in our internal controls over financial reporting that are deemed to be material weaknesses or that may require prospective or retroactive changes to our financial statements or identify other areas for further attention or improvement. If we identify one or more material weaknesses or determine we have inadequate internal controls, it could result in an adverse reaction in the financial markets due to a loss of confidence in the reliability of our financial statements.

If securities or industry analysts cease to publish research or reports about our business, or if they issue an adverse or misleading opinion regarding our ordinary shares, our share price and trading volume could decline.

The trading market for our ordinary shares relies in part on the research and reports that industry or securities analysts publish about us or our business. We do not control these analysts. Furthermore, if any of the analysts who cover us issue an adverse or misleading opinion regarding us, our business model, our intellectual property or our share performance, or if any of our preclinical studies or clinical trials and operating results fail to meet the expectations of analysts, our share price would likely decline. If one or more of these analysts ceases coverage of us or fails to publish reports on us regularly, we could lose visibility in the financial markets, which in turn could cause our share price or trading volume to decline.

Expectations relating to environmental, social and governance factors may impose additional costs and expose us to new risks.

There has been a focus in recent years by the SEC, foreign regulators, stock exchanges, certain investors and other stakeholders concerning corporate responsibility, specifically related to environmental, social and governance factors. Although the SEC adopted climate-related disclosure rules in 2024, those rules were stayed pending litigation and, in May 2026, the SEC proposed rescinding the rules in their entirety. Corporate sustainability rules in Europe also have been scaled back. As a result, the regulatory environment relating to climate-related disclosures remains subject to significant uncertainty. Nevertheless, some investors may use these and other environmental, social and governance factors to guide their investment strategies and, in some cases, may choose not to invest in us if they believe our policies and disclosures relating to corporate responsibility are inadequate. Third-party providers of corporate responsibility ratings and reports on companies have varied and in some cases inconsistent standards. In addition, the criteria by which companies' corporate responsibility practices are assessed are evolving, which could result in greater expectations of us and cause us to undertake costly initiatives to satisfy such new criteria. Alternatively, if we elect not to or are unable to satisfy such new criteria or do not meet the criteria of a specific third-party provider, some investors may conclude that our policies with respect to corporate responsibility are insufficient. We may face reputational damage in the event that our corporate responsibility procedures or standards do not meet the standards set by various constituencies. Furthermore, if our competitors' corporate responsibility performance is perceived to be greater than ours, potential or current investors may elect to invest with our competitors instead. In addition, in the event that we communicate or disclose certain initiatives and goals regarding environmental, social and governance matters, we could fail, or be perceived to fail, in our achievement of such initiatives or goals, or we could be criticized for the scope of such initiatives or goals or be subject to litigation for such failures. If we fail to satisfy the expectations of investors and other

stakeholders or our initiatives are not executed as planned, our reputation and financial results could be adversely affected.

Because we do not anticipate paying any cash dividends on our ordinary shares in the foreseeable future, capital appreciation, if any, would be your sole source of gain.

Under Cayman Islands law, we may only make distributions by way of dividend out of profits, or out of our share premium account (provided that immediately following the date that the dividend is proposed to be paid we are able to pay our debts as they fall due in the ordinary course of business). We have never declared or paid any cash dividends on our ordinary shares. We currently anticipate that we will retain future earnings for the development, operation and expansion of our business and do not anticipate declaring or paying any cash dividends for the foreseeable future. In addition, the Royalty Note Purchase Agreement prohibits us from paying cash dividends during its term and the terms of existing and future financing agreements may also preclude us from paying dividends. As a result, capital appreciation, if any, of our ordinary shares would be your sole source of gain on an investment in our ordinary shares for the foreseeable future. See the "Dividend Policy" section of our Form 10-K for the year ended December 31, 2025 previously filed with the SEC for additional information.

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.

On September 23, 2026, Keith R. Harris, Ph.D., the chairman of our Board of Directors, adopted a Rule 10b5-1 trading arrangement that is intended to satisfy the affirmative defense of Rule 10b5-1(c) for the sale of up to 40,000 of the Company's ordinary shares until July 13, 2027.

Item 6. Exhibits.

Exhibit Number	Description	Form	File No.	Exhibit	Filing Date	Filed/Furnished Herewith
10.1†	Amendment to the Collaboration and License Agreement and Device Assignment and Assumption Agreement, dated September 29, 2026, by and among Reogen Limited (formerly known as Hologen Neuro AI Limited), Hologen Neuro AI UK Limited, Hologen Limited, MeiraGTx Holdings plc, MeiraGTx Neuro UK Limited, MeiraGTx Neuro I, LLC and MeiraGTx, LLC.					*
10.2#	Non-Employee Director Compensation Program.					*
31.1	Certification of Principal Executive Officer Pursuant to Rules 13a-14(a) and 15d-14(a) under the Securities Exchange Act of 1934, as Adopted Pursuant to Section 302 of the Sarbanes-Oxley Act of 2002.					*
31.2	Certification of Principal Financial Officer Pursuant to Rules 13a-14(a) and 15d-14(a) under the Securities Exchange Act of 1934, as Adopted Pursuant to Section 302 of the Sarbanes-Oxley Act of 2002.					*
32.1	Certification of Principal Executive Officer Pursuant to 18 U.S.C. Section 1350, as Adopted Pursuant to Section 906 of the Sarbanes-Oxley Act of 2002.					**
32.2	Certification of 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.INS	Inline XBRL Instance Document					*
101.SCH	Inline XBRL Taxonomy Extension Schema Document					*
101.CAL	Inline XBRL Taxonomy Extension Calculation Linkbase Document					*
101.DEF	Inline XBRL Taxonomy Extension Definition Linkbase Document					*
101.LAB	Inline XBRL Taxonomy Extension Label Linkbase Document					*
101.PRE	Inline XBRL Taxonomy Extension Presentation Linkbase Document					*
104	Cover Page Interactive Data File (Formatted in Inline XBRL and contained in exhibit 101)					*

* Filed herewith.
** Furnished herewith.
# Management contract or compensation plan or arrangement.
† Portions of this exhibit (indicated by asterisks) have been omitted pursuant to Item 601(b)(10)(iv) of Regulation S-K.

SIGNATURES

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

MeiraGTx Holdings plc (Registrant)

Date: November 12, 2026

By: /s/ Alexandria Forbes

Alexandria Forbes
Chief Executive Officer
(*principal executive officer and authorized signatory*)

Date: November 12, 2026

By: /s/ Richard Giroux

Richard Giroux
Chief Financial Officer and Chief Operating Officer
(*principal financial officer and principal accounting officer*)

Certain information marked as [***] has been excluded from this exhibit because it is both (i) not material and (ii) is the type that the registrant treats as private or confidential.

AMENDMENT TO THE COLLABORATION AND LICENSE AGREEMENT AND DEVICE ASSIGNMENT AND ASSUMPTION AGREEMENT

This Amendment to the Collaboration and License Agreement and Device Assignment and Assumption Agreement (this "Agreement") is dated September 29, 2026 (the "Effective Date") and is made by and among MeiraGTx, LLC, a Delaware limited liability company ("MeiraGTx US"), MeiraGTx Neuro I, LLC, a Delaware limited liability company ("MeiraGTx Neuro US"), MeiraGTx Neuro UK Limited, a company incorporated in England with company number 16108121 ("MeiraGTx Neuro UK"), MeiraGTx Holdings plc, a Cayman Islands exempted company ("MeiraGTx Holdings", and together with MeiraGTx US, MeiraGTx Neuro US and MeiraGTx Neuro UK, "MeiraGTx"), Reogen Limited (formerly Hologen Neuro AI Limited), a Guernsey company with company number 74942 ("Reogen"), Hologen Neuro AI UK Limited, a company incorporated in England with company number 16283812 ("Hologen UK"), and Hologen Limited, a Guernsey company with company number 74905 ("HGL", and together with Reogen and Hologen UK, the "Company"). References in this Agreement to MeiraGTx or the Company include each of the entities so defined, jointly and severally. Each of the above is a "Party" and together they are the "Parties."

BACKGROUND

WHEREAS, under the Collaboration and License Agreement, dated April 20, 2026, by and among MeiraGTx Neuro US, the Company and the other entities party thereto (the "Collaboration Agreement"), MeiraGTx Neuro US granted Reogen and Hologen UK a license to Develop, Manufacture and Commercialize the Licensed Device in the Field in the Territory, and retained the rights set out in Section 2.3.2(b) of the Collaboration Agreement;

WHEREAS, the Parties have agreed that the Assigned IP and the Assigned Contracts relating to the Licensed Device will be assigned to Reogen;

WHEREAS, Hologen UK is a party to this Agreement because the license to the Licensed Device granted at Section 2.1.1(c) of the Collaboration Agreement is granted to both Reogen and Hologen UK, and both are required to give the acknowledgments in Section 6;

WHEREAS, HGL, MeiraGTx Neuro UK and MeiraGTx Holdings are the other parties to the Collaboration Agreement and are party to this Agreement so that Section 6 takes effect as an amendment of the Collaboration Agreement; and

WHEREAS, the Parties intend that, from the Effective Date, Reogen will own the Device outright and the rights of MeiraGTx Neuro US and its relevant Affiliates in respect of the Device will be limited to those expressly set out in Sections 3, 4 and 6.

NOW, THEREFORE, in consideration of the mutual covenants and agreements contained herein and other good and valuable consideration, including Reogen's payment of the Device Development Invoices under Section 2.7, the sufficiency of which is hereby acknowledged, the Parties hereby agree as follows:

1. DEFINITIONS

Capitalized terms not defined herein have the meanings given in the Collaboration Agreement, and where a provision of the Collaboration Agreement is cited in this Agreement, "MeiraGTx" in that provision has the meaning given in the Collaboration Agreement. "Assigned Patents" means the patents and patent applications listed in Schedule 1, together with all patents issuing from them, and all continuations, divisionals, continuations-in-part, reissues, reexaminations and foreign counterparts. "Assigned IP" means (a) the Assigned Patents, (b) the Assigned Know-How, and (c) all other intellectual property rights owned by MeiraGTx Neuro US or any of its relevant Affiliates as of the Effective Date that relate specifically to the Device or its Development, Manufacture or use, including rights in designs, drawings, specifications, software and firmware, copyrights, and the data, reports and other content relating to the Device. "Assigned Know-How" has the meaning given in Section 2.6. "Assigned Contracts" means the contracts listed in Schedule 2. "Device Development Invoices" means the invoices listed in Schedule 3. "Device" means the Licensed Device. "Post-Effective Date Invoice" has the meaning given in Section 2.7. "Licensed Product" has the meaning given in the Collaboration Agreement, and for clarity the Licensed Products include the Existing AAV-GAD Product, the Existing AAV-BDNF Product and the Additional Licensed Products. "GAD Product" means the Licensed Product described in clause (a) of the definition of "Licensed Product" in the Collaboration Agreement. "Reversion Products" means the GAD Product and the Existing AAV-BDNF Product. "Milestone" means (i) for the GAD Product, the date on which treatment of the last patient in a pivotal Phase III Clinical Trial of the GAD Product that is designed to support an application for Marketing Approval is complete, as reported to the DB; and (ii) for the Existing AAV-BDNF Product, the date on which treatment of the last patient in a pivotal Phase III Clinical Trial of the Existing AAV-BDNF Product that is designed to support an application for Marketing Approval is complete, as reported to the DB. "Improvements" means all improvements and modifications to the Device made by or for the Company or its Affiliates after the Effective Date, and all patents and patent applications claiming them, in each case only to the extent owned solely by the Company or its Affiliates, and excluding MeiraGTx Arising IP and Joint Arising IP. "Reversion Event" has the meaning given in Section 4.1. "Framework Agreement" means the framework agreement dated March 9, 2025 among HGL, Reogen, MeiraGTx Neuro UK and MeiraGTx Holdings, as amended from time to time.

2. ASSIGNMENT AND ASSUMPTION

2.1 **Assigned IP.** MeiraGTx Neuro US assigns, and agrees to assign, and will cause each of its relevant Affiliates to assign, to Reogen all of its and their rights, titles and interests in the Assigned IP, including the right to sue for past infringement and, subject to the Collaboration Agreement and Section 3.1 of this Agreement, the exclusive right to determine the further Development of, and the regulatory strategy for, the Device (save for any IND already filed by MeiraGTx as of the Effective Date), and Reogen accepts the assignment of all of MeiraGTx Neuro US's and its relevant Affiliates' rights, titles and interests in the Assigned IP and, after the Effective Date, will be responsible at its own cost for the prosecution, maintenance Development, Manufacture and Commercialization of the Assigned IP. MeiraGTx shall have no further responsibility to fund the prosecution, maintenance, Development, Manufacture or Commercialization of the Assigned IP after the Effective Date. For the avoidance of doubt, the assignment is not limited to the Field or to any Territory.

2.2 **Contracts.** MeiraGTx US assigns, and agrees to assign, and will cause each of its relevant Affiliates to assign, all of its and their rights, titles and interests in the Assigned Contracts to Reogen and delegates its obligations under them, and Reogen assumes and will perform all obligations, responsibilities and duties under them arising on or after the Effective Date. MeiraGTx US remains responsible for all obligations and liabilities under the Assigned Contracts arising before the Effective Date, other than the Device Development Invoices, and will indemnify Reogen against them. Reogen agrees to indemnify MeiraGTx US from and against all obligations and liabilities

under the Assigned Contracts arising on or after the Effective Date. Notwithstanding the foregoing in this Section 2.2, Reogen agrees to pay the Post-Effective Date Invoice as set forth in Section 2.7 to this Agreement.

2.3 **Consents.** Where an Assigned Contract requires the counterparty's consent to assignment, MeiraGTx US or the relevant Affiliate will [***] obtain such consent [***] after the Effective Date and Reogen will give the cooperation and information the counterparty reasonably requests. Until a required consent is obtained, MeiraGTx US or the relevant Affiliate will hold that contract for Reogen's benefit and Reogen will perform and pay for it as if it had been assigned. During that period MeiraGTx US or the relevant Affiliate will exercise its rights under that contract only as Reogen directs, will pass to Reogen all benefits it receives under it, and will not amend, terminate or waive any right under it without Reogen's prior written consent.

2.4 **Further assurance.** Each of MeiraGTx US and MeiraGTx Neuro US will, at Reogen's reasonable request, execute short-form assignments and any other instruments, provide documents and information in its possession, and take any other action Reogen reasonably requests in order to record, perfect and give full effect to Reogen's ownership of the Assigned IP in any jurisdiction, including responding to reasonable requests from any patent office.

2.5 **Condition of the assets.** The Assigned IP and the Assigned Contracts are assigned "as is," and MeiraGTx Neuro US, MeiraGTx US and the relevant Affiliates make no representation or warranty of any kind in respect of the Assigned IP and Assigned Contracts, as applicable, express or implied, other than MeiraGTx representing and warranting to Reogen as of the Effective Date that: (a) Schedule 1 is complete and comprehensive, including any patent applications, continuations, divisionals, continuations-in-part, reissues, reexaminations or foreign counterparts; (b) each inventor named in the Assigned Patents has assigned all of his or her rights in them to MeiraGTx Neuro US (directly or through a predecessor in title), and no compensation is payable to any inventor in respect of them; (c) MeiraGTx Neuro US is the sole owner of the Assigned Patents, and, to MeiraGTx's knowledge, MeiraGTx Neuro US and its relevant Affiliates are the sole owners of the other Assigned IP, in each case free of any lien, security interest, license or other encumbrance, other than the licenses granted under the Collaboration Agreement and the rights described in clause (d); (d) neither MeiraGTx Neuro US nor any of its relevant Affiliates has granted any third party any right under the Assigned IP, other than under the Collaboration Agreement and non-exclusive rights granted to service providers under the Assigned Contracts for the purposes of those contracts; and (e) each Assigned Contract is in full force and effect, MeiraGTx US or its relevant Affiliate has given Reogen a true and complete copy of it, and neither MeiraGTx US nor any of the relevant Affiliates nor, to MeiraGTx US's knowledge, the counterparty, is in material breach of such Assigned Contract. Nothing in this Agreement assigns or licenses any right held by MeiraGTx under any agreement with a third party, other than the Assigned Contracts.

2.6 **Know-How and Copyrights.** MeiraGTx Neuro US assigns and delivers, and agrees to assign and deliver, and will cause each of its relevant Affiliates to assign and deliver, to Reogen all of its and their right, title and interest in the Device-specific Know-How and copyrights owned by MeiraGTx or any of its Affiliates that are in the possession or control of MeiraGTx Neuro US or any of its relevant Affiliates as of the Effective Date (the "Assigned Know-How"), including the design history file, Device Master File, technical documentation, specifications, software and firmware, and manufacturing and quality records, and the content relating to the Device contained in any Regulatory Document. MeiraGTx will respond to Reogen's reasonable requests for specific items of Assigned Know-How.

2.7 **Consideration.** On or before the Effective Date, Reogen will pay the Device Development Invoices, [***] by wire transfer to the account MeiraGTx US designates in writing. Subsequent to the Effective Date, upon being presented with an invoice for work completed [***], Reogen will

pay the invoiced amount by wire transfer to the account MeiraGTx US designates in writing (the "Post-Effective Date Invoice"). The foregoing payments are the entire, full and final consideration payable for the assignments under this Section 2. Each of MeiraGTx US and MeiraGTx Neuro US, for itself and its relevant Affiliates, confirms that, except as set forth in this Section 2.7, no other amount is payable by the Company or its Affiliates to any of MeiraGTx US, MeiraGTx Neuro US or their relevant Affiliates in respect of the Development of the Device before the Effective Date.

2.8 **Closing.** The assignments under this Section 2 take effect on the Effective Date, simultaneously with the payment of invoices [***] under Section 2.7, and neither takes effect without the other.

3. COVENANTS

3.1 **Development of Device**. Reogen will use Commercially Reasonable Efforts to Develop the Device as needed for the Development of the Licensed Products. MeiraGTx's remedies for a breach of this Section 3.1 are limited to damages, specific performance and, where Section 4.1(b) applies, the Reversion License, and a breach of this Section 3.1 does not give rise to any right to a reassignment of the Assigned IP back to MeiraGTx or to terminate the Collaboration Agreement.

3.2 **Preservation of the Device**. Without the prior written consent of MeiraGTx, which consent shall not be unreasonably withheld, conditioned or delayed, Reogen will not, and will cause its Affiliates not to, sell, exclusively license, abandon, or otherwise transfer or encumber any Assigned IP, Assigned Contract or Know-How assigned under Section 2.6, or cease to Manufacture the Device, in each case in any manner that would remove or compromise the practical or commercially reasonable availability of the Device for any Reversion Product, or would result in consideration that is below the commercially reasonable fair market value of the Device. Reogen will cause any permitted transferee to execute a joinder agreeing to be bound by Sections 3, 4 and 6.3 of this Agreement.

4. REVERSION LICENSE

4.1 **Reversion Events.** Each of the following is a "Reversion Event":

(a) termination of the Collaboration Agreement in circumstances giving MeiraGTx rights under Section 13.3.4 of the Collaboration Agreement, or by MeiraGTx under Section 13.2.3 of the Collaboration Agreement; or

(b) Reogen's material failure to use Commercially Reasonable Efforts to Develop the Device as required under Section 3.1 of this Agreement, continuing for [***] after MeiraGTx's written notice specifying the failure in reasonable detail; provided that Reogen will provide MeiraGTx with reasonably detailed written updates on its Development activities under Section 3.1 annually, to give MeiraGTx a factual basis for assessing compliance with this Section 4.1(b).

No Reversion Event occurs while the Company disputes in good faith (i) a termination of the Collaboration Agreement, in accordance with Section 13.2.2 of the Collaboration Agreement, or (ii) the existence of, or the failure to cure, a failure under Section 4.1(b), in accordance with Section 7.3 of this Agreement, unless and until that dispute is resolved in favor of MeiraGTx; provided that during the pendency of any such dispute, all the terms and conditions of this Agreement will remain in effect and the Parties will continue to perform all of their respective obligations hereunder.

4.2 **Reversion License.** Reogen hereby grants, exercisable from a Reversion Event, to MeiraGTx Neuro US a non-exclusive, royalty-free, fully paid-up, perpetual license under the Assigned IP and the Improvements to Develop, Manufacture, import, supply and use the Device for the Development, Manufacture and Commercialization of the Reversion Products (the "Reversion

License"), with the right to sublicense through multiple tiers to its Affiliates and to third parties for the Development, Manufacture and use of the Device with the Reversion Products. The Reversion License does not include any right to sell, supply or license the Device on a standalone basis, or for use with any product that is not a Reversion Product. If a Reversion Event occurs after the Milestone has been achieved for either the GAD Product or the Existing AAV-BDNF Product, then MeiraGTx shall pay Reogen a one-time consideration equal to [***], and the Reversion License takes effect only upon such payment. MeiraGTx may assign the Reversion License, in whole or for any Reversion Product, to an Affiliate or to any acquirer or licensee of that Reversion Product. On a Reversion Event, Reogen will promptly deliver to MeiraGTx copies of the design history file, Device Master File, specifications, manufacturing records for the Device and the content relating to the Device contained in any Regulatory Document, for use only within the scope of the Reversion License. The Reversion License binds any successor owner of the Assigned IP or the Improvements, and is a license of "intellectual property" for the purposes of Section 365(n) of the U.S. Bankruptcy Code.

5. LIABILITY

5.1 Except in respect of a breach of Sections 2.2 and 7.1 of this Agreement, no Party is liable for any indirect, special, incidental, exemplary, punitive or consequential damages arising out of this Agreement.

5.2 Each Party may enforce Sections 2, 3, 4 and 6 of this Agreement by injunction or specific performance without the need to prove damage or post security, in addition to any other remedy available to it.

6. THE COLLABORATION AGREEMENT

6.1 **The Assigned IP.** The Company acknowledges and agrees that from the Effective Date the license granted at Section 2.1.1(c) of the Collaboration Agreement does not extend to the Assigned IP, that Reogen's rights in the Assigned IP derive solely from Section 2 of this Agreement and are subject to Section 4 of this Agreement, and that neither Reogen nor Hologen UK will exercise, or assert against MeiraGTx, that license in respect of the Assigned IP. Nothing in this Section 6.1 limits that license in respect of any MeiraGTx Licensed Technology other than the Assigned IP.

6.2 **Effect on the Collaboration Agreement.** Except as set out in this Section 6, the Collaboration Agreement continues in full force and effect. With effect from the Effective Date, and notwithstanding anything in the Collaboration Agreement: (a) the Assigned IP ceases to be MeiraGTx Licensed Technology, and the rights retained by MeiraGTx under Section 2.3.2(b)(ii) of the Collaboration Agreement cease to apply to the Device and the Assigned IP; (b) Sections 13.3.1(b) and 13.3.4 of the Collaboration Agreement do not apply to the Device, the Assigned IP, the Assigned Contracts or the Improvements, and the rights of MeiraGTx in respect of them on any expiry or termination of the Collaboration Agreement are solely those set out in Section 4 of this Agreement; (c) Section 13.3.6 of the Collaboration Agreement applies to Regulatory Documents and Regulatory Approvals for the Device only by way of the right of reference described in that Section, and only for the purposes of the Reversion License; (d) Section 4.4(b) of the Collaboration Agreement ceases to apply, subject to Sections 3.2, 4.1 and 4.2 of this Agreement; and (e) Section 5.2 of the Collaboration Agreement does not apply to the Device and Reogen's obligations in respect of the Development of the Device, subject to Sections 4.1 and 4.2 of this Agreement, are solely those set out in Section 3.1 of this Agreement. This Section 6 is an amendment of the Collaboration Agreement for the purposes of Section 14.2 of the Collaboration Agreement, and prevails over the Collaboration Agreement to the extent of any conflict.

6.3 **License for Collaboration Activities.** Reogen grants to MeiraGTx and its Affiliates a non-exclusive, non-transferable, royalty-free license under the Assigned IP and the Improvements, without the right to sublicense other than as reasonably necessary to permitted Subcontractors, solely to the extent necessary to perform the activities allocated to MeiraGTx under and in accordance with the Collaboration Agreement, including to sponsor and conduct any Clinical Trial of any Licensed Product in accordance with Section 3.7 of the Collaboration Agreement. This license does not extend to any use of the Device in connection with any product or program other than the Licensed Products, and ends on the expiry or termination of the Collaboration Agreement.

7. GENERAL

7.1 **Confidentiality.** Each Party will keep confidential, and will not use other than for the purposes of this Agreement, the terms of this Agreement and all confidential or proprietary information disclosed to it by another Party in connection with it, except to the extent the information is or becomes public other than by breach of this Section, was known to the receiving Party free of any obligation of confidence, or is independently developed by it. A Party may disclose such information to its Affiliates and to its and their directors, officers, employees, advisers, auditors and financing sources who are bound by obligations of confidence in order to further the purposes of this Agreement and the Collaboration Agreement. A Party may also disclose such information as required by law, court order or the rules of any securities exchange, in which case it will give the disclosing Party reasonable prior notice where lawful and practicable and use reasonable efforts to secure confidential treatment of such confidential or proprietary information (whether through protective order or otherwise).

7.2 **Governing law.** This Agreement and any dispute arising out of it are governed by the laws of the State of New York, without regard to its conflicts of law principles.

7.3 **Disputes.** If a dispute arises under this Agreement, the Parties will discuss it in good faith, including at least one discussion between senior executives of each Party. If it is not resolved within [***] of a written request for discussions, any Party may pursue the matter in a court of competent jurisdiction, and each Party submits to the exclusive jurisdiction of the state and federal courts sitting in the Borough of Manhattan, New York, New York.

7.4 **Notices.** Notices must be in writing and delivered by e-mail, nationally or internationally recognized overnight courier, or by registered or certified mail with postage prepaid and return receipt requested, to the address below or any address a Party notifies to the others. Notice is effective on receipt.

To MeiraGTx US, MeiraGTx Neuro US, MeiraGTx Neuro UK or MeiraGTx Holdings: [***].

To Reogen or HGL: [***].

To Hologen UK: [***].

7.5 **Assignment.** Except as provided in this Section 7.5, no Party may assign this Agreement without the prior written consent of the other Parties hereto. Subject to Section 3.2 of this Agreement, any Party may assign this Agreement to an Affiliate or to a successor in interest to the business or assets to which this Agreement relates, and will remain liable for the performance of any Affiliate assignee. Any assignment of this Agreement by MeiraGTx US or MeiraGTx Neuro US carries the license under Section 4.2 only in accordance with, and subject to, that Section.

7.6 **Entire agreement and amendment.** This Agreement, together with its Schedules, is the entire agreement among the Parties on its subject matter and supersedes all prior communications on that

subject matter; provided that, except as set out in Section 6, nothing in this Agreement supersedes or amends the Collaboration Agreement or the Framework Agreement. It may be amended only in writing signed by all Parties, and no waiver is effective unless in writing signed by the Party giving it.

7.7 **Miscellaneous.** This Agreement may be executed in counterparts, each of which is an original and all of which together are one instrument, and delivery of an executed counterpart by electronic means is effective. If any provision is held unenforceable, the remainder continues in effect. Nothing in this Agreement confers any right on any person other than the Parties. Headings are for convenience only. Schedules 1, 2 and 3 form part of this Agreement.

[SIGNATURE PAGE FOLLOWS]

IN WITNESS WHEREOF, the Parties have caused this Agreement to be executed by their duly authorized representatives as of the Effective Date.

MEIRAGTX, LLC

By: /s/ Richard Giroux
Name: Richard Giroux
Title: COO

MEIRAGTX NEURO I, LLC

By: /s/ Richard Giroux
Name: Richard Giroux
Title: CFO

MEIRAGTX NEURO UK LIMITED

By: /s/ Richard Giroux
Name: Richard Giroux
Title: CFO

MEIRAGTX HOLDINGS PLC

By: /s/ Richard Giroux
Name: Richard Giroux
Title: CFO and COO

REOGEN LIMITED

By: /s/ Andrew Henton
Name: Andrew Henton
Title: Director

HOLOGEN LIMITED

By: /s/ Andrew Henton
Name: Andrew Henton
Title: Director

HOLOGEN NEURO AI UK LIMITED

By: /s/ Parashkev Nachev
Name: Parashkev Nachev
Title: Director

MEIRAGTX HOLDINGS PLC

NON-EMPLOYEE DIRECTOR COMPENSATION PROGRAM
(EFFECTIVE AS OF SEPTEMBER 22, 2026)

Non-employee members of the board of directors (the "***Board***") of MeiraGTx Holdings plc (the "***Company***") shall receive cash and equity compensation as set forth in this Non-Employee Director Compensation Program (this "***Program***"). The cash and equity compensation described in this Program shall be paid or be made, as applicable, automatically and without further action of the Board, to each member of the Board who is not an employee of the Company or any parent or subsidiary of the Company (each, a "***Non-Employee Director***") who is entitled to receive such cash or equity compensation, unless such Non-Employee Director declines the receipt of such cash or equity compensation by written notice to the Company. This Program shall remain in effect until it is revised or rescinded by further action of the Board. This Program may be amended, modified or terminated by the Board at any time in its sole discretion. As of its effectiveness, the terms and conditions of this Program shall supersede any prior cash and/or equity compensation arrangements for service as a member of the Board between the Company and any of its Non-Employee Directors.

I. CASH COMPENSATION

A. Annual Retainers. Each Non-Employee Director shall receive an annual retainer of $90,000 for service on the Board.

B. Additional Annual Retainers. In addition, each Non-Employee Director shall receive the following annual retainers:

1. *Chairman of the Board or Lead Independent Director*. A Non-Employee Director serving as Chairman of the Board or Lead Independent Director shall receive an additional annual retainer of $75,000 for such service.

2. *Audit Committee*. A Non-Employee Director serving as Chairperson of the Audit Committee shall receive an additional annual retainer of $40,000 for such service. A Non-Employee Director serving as a member other than the Chairperson of the Audit Committee shall receive an additional annual retainer of $20,000 for such service.

3. *Compensation Committee*. A Non-Employee Director serving as Chairperson of the Compensation Committee shall receive an additional annual retainer of $30,000 for such service. A Non-Employee Director serving as a member other than the Chairperson of the Compensation Committee shall receive an additional annual retainer of $15,000 for such service.

4. *Nominating and Corporate Governance Committee*. A Non-Employee Director serving as Chairperson of the Nominating and Corporate Governance Committee shall receive an additional annual retainer of $30,000 for such service. A Non-Employee Director serving as a member other than the Chairperson of the Nominating and

Corporate Governance Committee shall receive an additional annual retainer of $15,000 for such service.

5. *Science and Technology Committee*. A Non-Employee Director serving as Chairperson of the Science and Technology Committee shall receive an additional annual retainer of $20,000 for such service. A Non-Employee Director serving as a member other than the Chairperson of the Science and Technology Committee shall receive an additional annual retainer of $10,000 for such service.

C. Payment of Retainers. The annual retainers described in Sections I(A) and I(B) shall be earned on a quarterly basis based on a calendar quarter and shall be paid in cash by the Company in arrears not later than the fifteenth day following the end of each calendar quarter. In the event a Non-Employee Director does not serve as a Non-Employee Director, or in the applicable positions described in Section I(B), for an entire calendar quarter, the retainer paid to such Non-Employee Director shall be prorated for the portion of such calendar quarter actually served as a Non-Employee Director, or in such position, as applicable.

II. EQUITY COMPENSATION

Non-Employee Directors shall be granted the equity awards described below. The awards described below shall be granted under and shall be subject to the terms and provisions of the Company's 2018 Incentive Award Plan or any other applicable Company equity incentive plan then-maintained by the Company (the "***Equity Plan***") and shall be granted subject to award agreements, including attached exhibits, in substantially the forms approved by the Board. All applicable terms of the Equity Plan apply to this Program as if fully set forth herein, and all grants of equity awards hereby are subject in all respects to the terms of the Equity Plan and the applicable award agreement.

A. Initial Awards. Each Non-Employee Director who is initially elected or appointed to the Board shall be granted 100,000 restricted share units on the date of such initial election or appointment. The awards described in this Section II(A) shall be referred to as "***Initial Awards***." No Non-Employee Director shall be granted more than one Initial Award.

B. Subsequent Awards. A Non-Employee Director who (i) has been serving as a Non-Employee Director on the Board for at least six months as of the date of any annual meeting of the Company's shareholders and (ii) will continue to serve as a Non-Employee Director immediately following such meeting, shall be automatically granted the following equity awards on the date of such annual meeting:

1. A Non-Employee Director serving as Chairman of the Board or Lead Independent Director shall be granted 100,000 restricted share units.

2. A Non-Employee Director serving as a member of the Board other than the Chairman or Lead Independent Director shall be granted 60,000 restricted share units.

The awards described in this Section II(B) shall be referred to as "***Subsequent Awards***." For the avoidance of doubt, a Non-Employee Director elected for the first time to the Board at an

annual meeting of the Company's shareholders shall only receive an Initial Award in connection with such election, and shall not receive any Subsequent Award on the date of such meeting as well.

C. Termination of Employment of Employee Directors. Members of the Board who are employees of the Company or any parent or subsidiary of the Company who subsequently terminate their employment with the Company and any parent or subsidiary of the Company and remain on the Board will not receive an Initial Award pursuant to Section II(A) above, but to the extent that they are otherwise entitled, will receive, after termination from employment with the Company and any parent or subsidiary of the Company, Subsequent Awards as described in Section II(B) above.

D. Terms of Awards Granted to Non-Employee Directors

1. *Vesting*. Each Initial Award shall vest in three equal annual installments, with one-third (1/3) of the award vesting on each of the first, second and third anniversaries of the grant date, such that the Initial Award shall be fully vested on the third anniversary of the date of grant, subject to the Non-Employee Director continuing in service as a Non-Employee Director through each such vesting date. Each Subsequent Award shall vest on the earlier of the first anniversary of the date of grant or the day immediately prior to the date of the next annual meeting of the Company's shareholders occurring after the date of grant, in either case subject to the Non-Employee Director continuing in service on the Board as a Non-Employee Director through each such vesting date. Unless the Board otherwise determines, any portion of an Initial Award or Subsequent Award which is unvested at the time of a Non-Employee Director's termination of service on the Board as a Non-Employee Director shall be immediately forfeited upon such termination of service and shall not thereafter become vested. All of a Non-Employee Director's Initial Awards and Subsequent Awards shall vest in full immediately prior to the occurrence of a Change in Control (as defined in the Equity Plan), to the extent outstanding at such time.

2. *Deferral*. The Board may provide that the settlement of restricted share units be deferred, on a mandatory basis or at the Non-Employee Director's election, on such terms as determined by the Board.

* * * * *

Exhibit 31.1

CERTIFICATION

I, Alexandria Forbes, certify that:

1. I have reviewed this Quarterly Report on Form 10-Q for the quarterly period ended September 30, 2026 of MeiraGTx Holdings plc;

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; and

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

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

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

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

Date: November 12, 2026

By: /s/ Alexandria Forbes

Alexandria Forbes
President and Chief Executive Officer
(*principal executive officer*)

Exhibit 31.2

CERTIFICATION

I, Richard Giroux, certify that:

1. I have reviewed this Quarterly Report on Form 10-Q for the quarterly period ended September 30, 2026 of MeiraGTx Holdings plc;

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; and

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

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

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

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

Date: November 12, 2026

By: /s/ Richard Giroux

Richard Giroux
Chief Financial Officer and Chief Operating Officer
(principal financial officer)

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 the Quarterly Report of MeiraGTx Holdings plc (the "Company") on Form 10-Q for the quarterly period ended September 30, 2026 as filed with the Securities and Exchange Commission on the date hereof (the "Report"), I certify, pursuant to 18 U.S.C. § 1350, as adopted pursuant to § 906 of the Sarbanes-Oxley Act of 2002, that to the best of my knowledge:

(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.

Date: November 12, 2026

By: /s/ Alexandria Forbes

Alexandria Forbes
President and Chief Executive Officer
(principal executive officer)

Exhibit 32.2

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 the Quarterly Report of MeiraGTx Holdings plc (the "Company") on Form 10-Q for the quarterly period ended September 30, 2026 as filed with the Securities and Exchange Commission on the date hereof (the "Report"), I certify, pursuant to 18 U.S.C. § 1350, as adopted pursuant to § 906 of the Sarbanes-Oxley Act of 2002, that to the best of my knowledge:

(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.

Date: November 12, 2026

By: /s/ Richard Giroux

Richard Giroux
Chief Financial Officer and Chief Operating Officer
(principal financial officer)