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UNITED STATES
SECURITIES AND EXCHANGE COMMISSION
WASHINGTON, D.C. 20549

FORM 10-Q

(Mark One)

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

For the quarterly period ended June 30, 2025

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.)**
450 East 29th Street, 14th Floor **New York, NY** **(Address of principal executive offices)**	**10016** **(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 July 31, 2025, the registrant had 80,447,477 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 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" 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.
- There is no guarantee that we will receive in a timely fashion or at all the additional milestone payments contemplated under the Asset Purchase Agreement or the revenues associated with our manufacture of the commercial supply of the RPGR Product under the Supply Agreement.
- 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, results of operations, and financial condition.

- 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 “Meira,” “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)

	June 30, 2025	December 31, 2024
ASSETS		
CURRENT ASSETS:		
Cash and cash equivalents	$ 32,166	$ 103,659
Accounts receivable - related party	2,319	707
Contract assets - related party	—	950
Inventory	314	385
Prepaid expenses	7,033	6,828
Tax incentive receivable	4,472	8,971
Other current assets	780	2,018
Total Current Assets	47,084	123,518
Property, plant and equipment, net	110,816	102,878
Intangible assets, net	742	821
Restricted cash	2,258	2,009
Other assets	1,093	1,002
Equity method and other investments	6,749	6,749
Right-of-use assets - operating leases, net	5,820	10,576
Right-of-use assets - finance leases, net	24,154	22,198
TOTAL ASSETS	$ 198,716	$ 269,751
LIABILITIES AND SHAREHOLDERS' EQUITY		
CURRENT LIABILITIES:		
Accounts payable	$ 25,586	$ 23,586
Accrued expenses	15,483	27,414
Lease obligations, current	2,647	4,053
Deferred revenue - related party, current	2,908	4,827
Other current liabilities	6,812	903
Total Current Liabilities	53,436	60,783
Deferred revenue - related party	63,044	57,576
Lease obligations	4,049	7,523
Asset retirement obligations	1,454	2,821
Note payable, net	73,773	73,221
TOTAL LIABILITIES	195,756	201,924
COMMITMENTS AND CONTINGENCIES (Note 11)		
SHAREHOLDERS' EQUITY:		
Ordinary Shares, $0.00003881 par value, 1,288,327,750 authorized, 80,446,284 and 78,397,380 shares issued and outstanding at June 30, 2025 and December 31, 2024, respectively	3	3
Capital in excess of par value	791,280	773,565
Accumulated other comprehensive loss	(7,525)	(3,719)
Accumulated deficit	(780,798)	(702,022)
Total Shareholders' Equity	2,960	67,827
TOTAL LIABILITIES AND SHAREHOLDERS' EQUITY	$ 198,716	$ 269,751

See Notes to Condensed Consolidated Financial Statements

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

	For the Three-Month Periods Ended June 30,		For the Six-Month Periods Ended June 30,	
	2025	2024	2025	2024
Revenues:				
Service revenue - related party	$ 3,691	$ 282	$ 5,617	$ 979
Total revenue	3,691	282	5,617	979
Operating expenses:				
Cost of service revenue - related party	2,676	—	4,054	—
General and administrative	12,313	11,257	21,677	24,404
Research and development	33,495	34,934	66,275	69,256
Total operating expenses	48,484	46,191	92,006	93,660
Loss from operations	(44,793)	(45,909)	(86,389)	(92,681)
Other non-operating income (expense):				
Foreign currency gain (loss)	8,624	(284)	12,311	(819)
Interest income	408	827	1,379	1,924
Interest expense	(3,034)	(3,254)	(6,077)	(6,504)
Gain on sale of nonfinancial assets	—	—	—	29,018
Net loss	(38,795)	(48,620)	(78,776)	(69,062)
Other comprehensive loss:				
Foreign currency translation loss	(2,459)	(488)	(3,806)	(2,179)
Comprehensive loss	$ (41,254)	$ (49,108)	$ (82,582)	$ (71,241)
Net loss	$ (38,795)	$ (48,620)	$ (78,776)	$ (69,062)
Basic and diluted net loss per ordinary share	$ (0.48)	$ (0.76)	$ (0.99)	$ (1.08)
Weighted-average number of ordinary shares outstanding	80,585,625	64,376,396	79,813,273	64,221,145

See Notes to Condensed Consolidated Financial Statements

MEIRAGTX HOLDINGS PLC AND SUBSIDIARIES
CONDENSED CONSOLIDATED STATEMENT OF SHAREHOLDERS' EQUITY
FOR THE PERIOD ENDED JUNE 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

See Notes to Condensed Consolidated Financial Statements

MEIRAGTX HOLDINGS PLC AND SUBSIDIARIES
CONDENSED CONSOLIDATED STATEMENT OF SHAREHOLDERS' EQUITY
FOR THE PERIOD ENDED JUNE 30, 2024
(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, 2023	63,601,015	$ 2	$ 693,841	$ (1,435)	$ (554,231)	$ 138,177
Share-based compensation activity	441,348	—	4,739	—	—	4,739
Issuance of ordinary shares in at-the-market offering	256,328	—	1,586	—	—	1,586
Issuance costs in connection with ordinary shares	—	—	(635)	—	—	(635)
Other comprehensive loss	—	—	—	(1,691)	—	(1,691)
Net loss for the three-month period ended March 31, 2024	—	—	—	—	(20,442)	(20,442)
Balance at March 31, 2024	64,298,691	2	699,531	(3,126)	(574,673)	121,734
Share-based compensation activity	50,000	—	5,810	—	—	5,810
Issuance of ordinary shares in at-the-market offering	335,496	1	1,611	—	—	1,612
Issuance costs in connection with ordinary shares	—	—	(9)	—	—	(9)
Other comprehensive loss	—	—	—	(488)	—	(488)
Net loss for the three-month period ended June 30, 2024	—	—	—	—	(48,620)	(48,620)
Balance at June 30, 2024	64,684,187	3	706,943	(3,614)	(623,293)	80,039

See Notes to Condensed Consolidated Financial Statements

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

	For the Six-Month Periods Ended June 30,	
	2025	2024
Cash flows from operating activities:		
Net loss	$ (78,776)	$ (69,062)
Adjustments to reconcile net loss to net cash used in operating activities:		
Share-based compensation expense	10,551	12,770
Foreign currency (gain) loss	(12,311)	819
Depreciation and amortization	6,271	6,497
Net change in right-of-use assets and liabilities	(138)	(100)
Gain on termination of lease liabilities	(1,365)	—
(Gain) loss on disposal of equipment, furniture and fixtures	(122)	434
Amortization of interest on asset retirement obligations	98	97
Amortization of debt discount	552	546
Gain on sale of nonfinancial assets	—	(29,018)
(Increase) decrease in operating assets:		
Accounts receivable - related party	(1,653)	7,764
Contract assets - related party	986	—
Inventory	122	—
Prepaid expenses	144	654
Tax incentive receivable	4,851	9,586
Other current assets	1,394	905
Other assets, net	—	632
Increase (decrease) in operating liabilities:		
Accounts payable	(2,968)	5,670
Accrued expenses	(12,595)	(24,928)
Other current liabilities	5,904	—
Deferred revenue - related party	(1,720)	20,537
Net cash used in operating activities	(80,775)	(56,197)
Cash flows from investing activities:		
Purchase of property, plant and equipment	(2,944)	(3,007)
Proceeds from sale of equipment, furniture and fixtures	473	—
Proceeds from sale of nonfinancial assets	—	29,018
Net cash provided by investing activities	(2,471)	26,011
Cash flows from financing activities:		
Exercise of share options	35	—
Payments of withholdings on shares withheld for income taxes	(2,761)	(2,221)
Proceeds from the issuance of ordinary shares	9,915	3,198
Issuance costs in connection with ordinary shares	(26)	(644)
Net cash provided by financing activities	7,163	333
Net decrease in cash, cash equivalents and restricted cash	(76,083)	(29,853)
Effect of exchange rate changes on cash, cash equivalents and restricted cash	4,839	229
Cash, cash equivalents and restricted cash at beginning of the period	105,668	130,649
Cash, cash equivalents and restricted cash at end of the period	$ 34,424	$ 101,025
Supplemental disclosure of non-cash transactions:		
Fixed asset acquisitions included in accounts payable and accrued expenses	$ 892	$ 1,181
Supplemental disclosure of cash flow information:		
Cash paid for interest	$ 5,557	$ 5,891

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 "Meira 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 late-stage clinical programs, including Parkinson's disease, radiation-induced xerostomia and AIPL1-associated retinal dystrophy. MeiraGTx clinical programs use targeted local delivery of small doses of genetic medicines to treat both inherited and more common conditions with severe unmet need. The successful development of the clinical pipeline is supported by the Company's internal end-to-end manufacturing capabilities. The Company has two viral vector production facilities for good manufacturing practices ("GMP"), internal plasmid production for GMP, as well as an in-house Quality Control hub for stability and release, all fit for IND through commercial supply. In addition, the Company has developed a proprietary manufacturing platform with leading yield and quality aspects and commercial readiness. The Company's core capabilities in viral vector and capsid optimization allow increased potency, decreased dose and significantly reduced cost of goods for its genetic medicines. The Company has developed a transformative gene regulation platform using bespoke synthetic riboswitch technology invented in-house that allows for the precise, dose-responsive expression of any transgene under the control of oral small molecules. The Company is focusing the riboswitch platform on *in vivo* delivery of biologic therapeutics such as the metabolic peptides GLP-1, GIP, glucagon, amylin, PYY and leptin via oral small molecules, as well as cell therapy for oncology and autoimmune diseases, and long-term intractable pain. The Company has developed unique comprehensive technology capabilities to apply genetic medicine to more common diseases, increasing efficacy, addressing novel targets, and expanding access in some of the largest disease areas where the unmet need remains high.

Hologen Transactions

On March 9, 2025 (the "Signing Date"), the Company and its affiliates entered into a strategic collaboration with Hologen Limited, a non-cellular company limited by shares incorporated in Guernsey ("Hologen"), and its affiliates. 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, the Company and Hologen have entered into the Framework Agreements (as defined below), pursuant to which the Company and its affiliates will receive from Hologen an upfront cash payment of $200 million (the "Upfront Payment") on the Closing Date (as defined below), and Hologen will provide additional funding of up to an additional $230 million as further described below. As part of the strategic collaboration, the Company also received 250,000 Class A shares of Hologen at a nominal price. Furthermore, the Company has the option to receive up to an additional 500,000 Class A shares of Hologen at a nominal price if certain funding obligations as described herein are not met by Hologen. During June 2025, Hologen made a $6.0 million payment as part of its commitment toward the Upfront Payment which is included in other current liabilities, and made an additional $17.0 million payment during the third quarter 2025.

The closing of the transactions contemplated by the Framework Agreements (the "Closing Date") is expected to occur in the third calendar quarter of 2025, subject to customary closing and funding conditions.

Neuro Framework Agreement

On the Signing Date, the Company, MeiraGTx Neuro UK Limited, a private company limited by shares incorporated in England and a wholly-owned subsidiary of the Company ("MeiraGTx Neuro UK"), Hologen Neuro AI Limited, a non-cellular company limited by shares incorporated in Guernsey and an affiliate of Hologen ("Hologen Neuro"), and Hologen, entered into that certain Framework Agreement (the "Neuro Framework Agreement"), pursuant to which, on the Closing Date, the Company, MeiraGTx Neuro UK, MeiraGTx Neuro I, LLC, a Delaware limited liability company and a wholly-owned subsidiary of the Company ("MeiraGTx Neuro US"), Hologen, Hologen Neuro and Hologen Neuro AI UK Limited, a private company limited by shares incorporated in England and an affiliate of Hologen ("Hologen Neuro UK"), shall enter into a Collaboration and License Agreement (the "Hologen Collaboration Agreement") for the research, development, manufacture and commercialization of the Company's (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 Hologen Collaboration Agreement.

On the Closing Date, under the Hologen Collaboration Agreement, MeiraGTx Neuro US will receive the applicable portion of the Upfront Payment in consideration for granting to Hologen Neuro and Hologen Neuro UK, as of the Closing Date and subject to the license granted by Hologen Neuro and Hologen Neuro UK back to MeiraGTx Neuro UK, exclusive, worldwide, royalty-free, fully paid-up licenses to certain of the Company's intellectual property rights for the research, development, manufacture and commercialization of the Clinical Programs and the Delivery Device. On the Closing Date, (a) MeiraGTx Neuro UK will receive Class A shares of Hologen Neuro representing a 30% ownership of the issued share capital of Hologen Neuro, in consideration for the provision of services to Hologen Neuro and Hologen Neuro UK as specified in the Hologen Collaboration Agreement, including services relating to the development of the Clinical Programs and the Delivery Device and certain other transition services, and (b) Hologen Guernsey will receive Class B shares of Hologen Neuro representing a 70% ownership of the issued share capital of Hologen Neuro, in consideration for paying the applicable portion of the Upfront Payment to MeiraGTx Neuro US, as well as a commitment to provide additional capital of up to $230 million to fund the development of the Clinical Programs and the Delivery Device. Additionally, Hologen will license to Hologen Neuro its proprietary multi-modal generative foundation models (LMMs), or large medicine models, pursuant to a license agreement mutually agreeable to the parties.

As of the Closing Date, Hologen Neuro shall be governed by a board of directors comprised of three representatives designated by Hologen and two representatives designated by MeiraGTx Neuro UK, and certain material business decisions (as further enumerated in the Neuro Framework Agreement) will require the approval of at least 70% of the directors then in office.

Following the Closing Date and in accordance with the terms of the Hologen Collaboration Agreement, the parties shall negotiate in good faith and enter into clinical and commercial supply agreements, pursuant to which MeiraGTx Neuro UK (directly, or through affiliates or subcontractors) shall manufacture and supply AAV-GAD, AAV-BDNF and other potential locally delivered genetic medicines to the central nervous system.

Manufacturing Framework Agreement

On the Signing Date, MeiraGTx Manufacturing Limited, a private company limited by shares incorporated in England and a wholly-owned subsidiary of the Company ("MeiraGTx Manufacturing"), MeiraGTx Limited, a private company limited by shares incorporated in England and a wholly-owned subsidiary of the Company ("MeiraGTx Limited"), and Hologen, entered into that certain Framework Agreement (the "Manufacturing Framework Agreement" and, together with the Neuro Framework Agreement, the "Framework Agreements"), pursuant to which, on the Closing Date and in exchange for the applicable portion of the Upfront Payment, Hologen will acquire a minority interest in MeiraGTx Manufacturing, an entity that will comprise the Company's flexible and scalable end-to-end genetic medicines manufacturing business, which is solely run by MeiraGTx Manufacturing and MeiraGTx Limited prior to the Closing Date. Hologen will also contribute a portion of the annual funding to MeiraGTx Manufacturing.

As of the Closing Date, MeiraGTx Manufacturing shall be governed by a board of directors comprised of three representatives designated by MeiraGTx Limited and two representatives designated by Hologen, and certain material business decisions (as further enumerated in the Manufacturing Framework Agreement) will require the approval of at least 70% of the directors then in office.

For a period of twelve months following the Closing Date, Hologen has an exclusive, irrevocable option to purchase additional shares in MeiraGTx Manufacturing at a specified price, such that following exercise of such option, Hologen shall own 40% of the issued share capital of MeiraGTx Manufacturing in the aggregate. In the event that Hologen does not exercise its option, MeiraGTx has an exclusive, irrevocable option to purchase all of the shares of MeiraGTx Manufacturing held by Hologen for the same price that Hologen paid for such shares. Such option shall be exercisable anytime beginning on the third anniversary of the Closing Date and ending three years thereafter.

Asset Purchase and Related Agreements with Johnson & Johnson Innovative Medicine

On January 30, 2019, the Company entered into a Collaboration, Option and License Agreement with Johnson & Johnson Innovative Medicine (formerly known as Janssen Pharmaceuticals, Inc.) (the "Collaboration Agreement"), for the research, development and commercialization of gene therapies for the treatment of inherited retinal diseases ("IRD"). Under the terms of the Collaboration Agreement, the Company received funding for certain research, manufacturing, clinical development and commercialization costs, and had the potential to obtain additional milestone payments upon the achievement of such milestones and royalties on future net sales of products. On December 20, 2023, the Company entered into an Asset Purchase Agreement ("Asset Purchase Agreement") with Johnson & Johnson Innovative Medicine pursuant to which the Company sold and assigned to Johnson & Johnson Innovative Medicine, and Johnson & Johnson Innovative Medicine purchased and assumed, that certain License Agreement, dated February 5, 2019, by and between UCL Business Plc (now UCL Business Ltd.) ("UCLB"), on the one hand, and MeiraGTx UK II Limited and MeiraGTx Limited, on the other hand (the "UCLB RPGR License Agreement"), relating to the research, development, manufacture and exploitation of botaretigene sparoparvovec, or bota-vec (formerly referred to as AAV-RPGR), for the treatment of X-linked retinitis pigmentosa related to mutations in the retinitis pigmentosa GTPase regulator gene, or XLRP-RPGR (the "RPGR Product"), and other related assets as described in the Asset Purchase Agreement. In connection with entering into the Asset Purchase Agreement, the Company entered into a Termination Agreement with Johnson & Johnson Innovative Medicine terminating the Collaboration Agreement. The Company and Johnson & Johnson Innovative Medicine also entered into a Supply Agreement on December 20, 2023 (the "Supply Agreement") pursuant to which the Company agreed to manufacture and supply the RPGR Product for Johnson & Johnson Innovative Medicine.

Under the Asset Purchase Agreement, Johnson & Johnson Innovative Medicine paid the Company a non-refundable upfront cash payment of $65.0 million in December 2023. Additionally, pursuant to and subject to the terms and conditions set forth in the Asset Purchase Agreement, Johnson & Johnson Innovative Medicine agreed to pay the Company future contingent consideration of up to an aggregate of $350.0 million, as follows: (i) a milestone payment of $50.0 million in connection with the achievement of the initiation of the extension study for the Phase 3 LUMEOS clinical trial for the RPGR Product; (ii) $10.0 million upon completion of certain specified development services for the drug substance for the RPGR Product; (iii) $5.0 million upon completion of certain specified development services for the drug product for the RPGR Product; (iv) $175.0 million upon the first commercial sale of an RPGR Product in the United States; (v) $75.0 million upon the first commercial sale of an RPGR Product in at least one of the United Kingdom, France, Germany, Spain and Italy; (vi) $25.0 million upon completion of the transfer of certain manufacturing technology for drug substance and drug product from the Company to Johnson & Johnson Innovative Medicine; and (vii) $10.0 million upon regulatory approval of a Johnson & Johnson Innovative Medicine-selected manufacturing facility in each of the United States and European Union for commercial manufacture of the RPGR Product. During 2024, the Company received $60.0 million in milestone payments from Johnson & Johnson Innovative Medicine. Johnson & Johnson Innovative Medicine is also responsible for any royalty or milestone amounts that become payable on the RPGR Product under the UCLB RPGR License Agreement.

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 six-month period ended June 30, 2025 are not necessarily indicative of the results that may be expected for the year ending December 31, 2025. 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, 2024 included in the Company's Annual Report on Form 10-K for the fiscal year ended December 31, 2024 (the "Form 10-K").

Liquidity

The Company has not yet achieved profitable operations. There is no assurance that profitable operations, if ever achieved, 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 June 30, 2025 totaled $780.8 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 six months ended June 30, 2025, the Company used $80.8 million in cash flows from operations and 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 June 30, 2025, the Company had cash, cash equivalents and restricted cash in the amount of $34.4 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, tax incentive receivable and accounts receivable – related party at June 30, 2025, together with the $17.0 million deposit received from Hologen during the third quarter 2025 and the remaining proceeds from the anticipated closing of the strategic collaboration with Hologen, 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.

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 Collaboration Agreement, Asset Purchase Agreement and private and public equity offerings, as well as the proceeds from the debt financing described in Note 10. 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, 2024 included in the Company's Form 10-K.

Consolidation

The accompanying condensed consolidated financial statements include the accounts of Meira Holdings and its wholly owned subsidiaries:

MeiraGTx Limited, a limited company incorporated under the laws of England and Wales;

MeiraGTx, LLC, a Delaware limited liability company ("Meira LLC");

MeiraGTx UK II Limited, a limited company incorporated under the laws of England and Wales ("Meira UK II");

MeiraGTx Ireland DAC, a designated activity company incorporated under the laws of Ireland ("Meira Ireland");

MeiraGTx Netherlands, B.V., a private company with limited liability incorporated under the laws of the Netherlands ("Meira Netherlands");

MeiraGTx Belgium, a private company with limited liability incorporated under the laws of Belgium ("Meira Belgium");

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 ("Meira B.V.");

MeiraGTx Neurosciences, Inc., a Delaware corporation;

MeiraGTx Therapeutics, Inc., a Delaware corporation;

MeiraGTx UK Limited, a limited company incorporated under the laws of England and Wales ("Meira UK");

MeiraGTx Neuro I, LLC, a Delaware limited liability company;

MeiraGTx Neuro II, LLC, a Delaware limited liability company;

MeiraGTx Manufacturing Limited, a limited company incorporated under the laws of England and Wales;

MeiraGTx Ocular UK Limited, a limited company incorporated under the laws of England and Wales;

MeiraGTx Gene Regulation Limited, a limited company incorporated under the laws of England and Wales;

MeiraGTx Neuro UK Limited, a limited company incorporated under the laws of England and Wales; and

MeiraGTx Cell Therapies, a simplified joint stock company, incorporated under the laws of France.

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 used significant estimates in the following areas, among others: service revenue, fair value of nonfinancial assets, stand-alone selling price and material rights in connection with the Asset Purchase and Supply Agreements, the accounting for research and development costs, share-based compensation, leases, asset retirement obligations, fair value of financial instruments and 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):

	June 30, 2025	December 31, 2024
Cash and cash equivalents	$ 32,166	$ 103,659
Restricted cash	2,258	2,009
Total cash, cash equivalents and restricted cash in the condensed consolidated statement of cash flows	$ 34,424	$ 105,668

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 Topic 820, *Fair Value Measurements and Disclosures*, or ASC 820, 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):

		Fair Value Measurement Using:		
Description	June 30, 2025	Significant Observable Inputs (Level 1)	Significant Other Observable Inputs (Level 2)	Significant Unobservable (Level 3)
Cash equivalents	$ 20,645	$ 20,645	$ —	$ —
Restricted cash	$ 2,258	$ 2,258	$ —	$ —

		Fair Value Measurement Using:		
Description	December 31, 2024	Significant Observable Inputs (Level 1)	Significant Other Observable Inputs (Level 2)	Significant Unobservable (Level 3)
Cash equivalents	$ 80,930	$ 80,930	$ —	$ —
Restricted cash	$ 2,009	$ 2,009	$ —	$ —

At June 30, 2025, the Company's financial instruments included cash and cash equivalents, restricted cash, accounts receivable – related party, and accounts payable. The carrying amounts reported in the Company's condensed consolidated financial statements for these instruments approximates their respective fair values because of the short-term nature of these instruments. In addition, at June 30, 2025, the Company believes the carrying value of the Tranche 1 Notes (as defined in Note 10) approximates fair value as the interest rate is reflective of the rate the Company could obtain on debt with similar terms and conditions.

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 interest expense in the condensed consolidated statements of operations and comprehensive loss.

During the three months ended March 31, 2025, the Company determined that it was reasonably certain to exercise its early termination option on an operating lease for laboratory and office space, for which the Company had an associated asset retirement obligation liability in the amount of $1.7 million and a corresponding leasehold improvement with a net book value of $0.3 million as of March 31, 2025. The landlord no longer requires the Company to make any changes to the premises prior to the lease termination and the Company only expects to incur $0.1 million related to decontamination costs. Therefore, a change in estimate of the asset retirement obligation was recorded resulting in a gain on termination of lease liabilities of $1.3 million which is included in general and administrative expenses in the accompanying condensed consolidated statements of operations.

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

	For the Six-Month Periods Ended June 30,	
	2025	2024
Balance at beginning of period	$ 2,821	$ 2,401
Change in estimate	(1,576)	—
Amortization of interest	98	97
Effects of exchange rate changes	111	(8)
Balance at end of period	$ 1,454	$ 2,490

IDA Ireland Grant

In August 2021, Meira 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, Meira 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"). Meira 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 sheet. All grant drawdowns 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. Grant income from the employment grant is recognized as a deduction from the amount of the related expense, and grant income from the capital grant is deducted from the carrying amount of the related asset and recognized in income over the asset's useful life in the form of a reduced depreciation charge. The Company received its first drawdown under the grant in 2023, which was comprised of $0.6 million for the employment grant and $0.4 million for the capital grant. The Company received its second drawdown under the grant in 2024, which was comprised of $0.5 million for the employment grant and $0.5 million for the capital grant. During the six-months ended June 30, 2025 and 2024, 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.

During the five-year period ending on the termination of the grant agreement, Meira 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 June 30, 2025, 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 unconditional. 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 sheet. Amounts expected to be recognized as revenue within the 12 months following the balance sheet date are classified as deferred revenue – related party, current. Amounts not expected to be recognized as revenue within the 12 months following the balance sheet date are classified as deferred revenue – related party.

The Company's revenue arrangements include the following:

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.

Research and Development Services: Under the Asset Purchase Agreement, research and development services (PPQ services) are recorded as incurred under cost of service revenue – related party.

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.

Net Loss per Ordinary Share

Basic net loss per ordinary share is computed by dividing net loss by the weighted average number of shares of the Company's ordinary shares assumed to be outstanding during the period of computation. Diluted net loss per ordinary share is computed similar to basic net loss per share except that the denominator is increased to include the number of additional ordinary shares that would have been outstanding if the potential ordinary share equivalents had been issued at the beginning of the year and if the additional ordinary shares were dilutive (treasury stock method) or the two-class method, whichever is more dilutive. For all periods presented, basic and diluted net loss per ordinary share are the same, as any additional ordinary share equivalents would be anti-dilutive.

The following securities are considered to be ordinary share equivalents, but were not included in the computation of diluted net loss per ordinary share because to do so would have been anti-dilutive:

	June 30, 2025	June 30, 2024
Share options	8,060,305	8,336,478
Restricted share units	6,252,250	4,357,250
Deferred share units	—	287,500
Warrants	700,000	700,000
	15,012,555	13,681,228

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, 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 chief operating decision maker ("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 that also is reported on the condensed consolidated statement of operations and comprehensive loss as net loss.

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 (in thousands):

	For the Three-Month Periods Ended June 30,		For the Six-Month Periods Ended June 30,	
	2025	2024	2025	2024
Service revenue - related party	$ 3,691	$ 282	$ 5,617	$ 979
Operating expenses:				
Cost of service revenue - related party	2,676	—	4,054	—
General and administrative	8,607	8,090	14,951	16,949
Clinical programs:				
Botaretigene sparoparvovec	—	816	—	1,135
AAV-hAQP1	2,754	4,617	7,606	6,279
AAV-CNGB3 / AAV-CNGA3	—	818	—	(361)
AAV-GAD	8,970	1,466	10,430	3,636
Other ocular diseases	20	—	1,465	293
Manufacturing	8,888	14,838	22,989	33,236
Preclinical programs:				
Gene regulation	3,241	3,101	5,527	5,600
Neurodegenerative diseases	298	361	724	751
Preclinical ocular diseases	1,162	583	1,787	910
Other research and development[1]	3,019	3,732	5,651	7,874
Johnson & Johnson Innovative Medicine reimbursement	—	(1,257)	—	(1,909)
Share-based compensation	5,659	5,806	10,551	12,770
Depreciation and amortization	3,190	3,220	6,271	6,497
Total operating expenses	48,484	46,191	92,006	93,660
Other segment items[2]	5,998	(2,711)	7,613	23,619
Segment net loss	$ (38,795)	(48,620)	$ (78,776)	(69,062)
Net loss	$ (38,795)	$ (48,620)	$ (78,776)	$ (69,062)

[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 gain (loss), interest income, interest expense and gain on sale of nonfinancial assets.

The Company's service revenue and deferred revenue from the Asset Purchase Agreement and related agreements were generated in the United Kingdom.

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

	June 30, 2025	December 31, 2024
United States	$ 2,494	$ 7,209
United Kingdom	28,725	28,419
European Union	110,313	100,845
	$ 141,532	$ 136,473

Recent Accounting Pronouncements Not Yet Adopted

In October 2023, the FASB issued ASU 2023-06, Disclosure Improvements: Codification Amendments in Response to the SEC's Disclosure Update and Simplification Initiative. This update includes a number of amendments to clarify or improve disclosure and presentation requirements of a variety of topics in order to allow users to more easily compare entities subject to the SEC's existing disclosures with those entities that were not previously subject to the requirements and to align the requirements in the FASB accounting standard codification with the SEC's regulations. The effective date for each amendment will be the date on which the SEC's removal of that related disclosure requirement from Regulation S-X or Regulation S-K becomes effective, with early adoption prohibited. The Company does not expect that the application of this standard will have an impact on its consolidated financial statements.

In December 2023, the FASB issued ASU 2023-09, Improvements to Income Tax Disclosures, which requires that an entity disclose specific categories in the effective tax rate reconciliation as well as provide additional information for reconciling items that meet a quantitative threshold. Further, the ASU requires certain disclosures of state versus federal income tax expense and taxes paid. This ASU is effective for annual periods beginning after December 15, 2024. The Company does not expect the adoption of this ASU to have a material impact on its consolidated financial statements.

In November 2024, the FASB issued ASU No. 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 No. 2025-01, Clarifying the Effective Date, which revised the effective date of ASU No. 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 consolidated financial statements.

In July 2025, the FASB issued ASU 2025-05, Financial Instruments - Credit Losses (Topic 326): Measurement of Credit Losses for Accounts Receivable and Contract Assets. This amendment introduces a practical expedient for the application of the current expected credit loss model to current accounts receivable and contract assets. The guidance is effective for annual periods beginning after December 15, 2025, and interim periods within those annual reporting periods. Early adoption is permitted. The Company is currently evaluating the timing of the adoption and the impact of this ASU on its consolidated financial statements and related disclosures.

3. Equity Method and Other Investments

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

		June 30, 2025		
Investee	**Investment Type**	**Ownership Percentage**	**Carrying Value**	**Cost Basis**
Visiogene LLC	Equity Method Investment	25.0 %	$ 5,133	$ 5,165
Other	Equity Investment	0.9 %	1,616	1,500
Total equity method and other investments			$ 6,749	$ 6,665

4. Accrued Expenses

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

	June 30, 2025	December 31, 2024
Professional fees	$ 4,925	$ 6,326
Research and development	3,254	2,234
Manufacturing costs	2,239	1,540
Clinical trial costs	1,879	3,864
Compensation and benefits	1,790	11,197
Consulting	1,055	1,530
Fixed assets	102	326
Rent and facilities costs	79	257
Other	160	140
	$ 15,483	$ 27,414

5. Share-Based Compensation

Equity Incentive Plans

The Company's 2018 Incentive Award Plan and 2016 Equity Incentive Plan (collectively, the "Plans") were adopted by the Company's board of directors and shareholders. Under the Plans, 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. The Company's board of directors or a committee thereof administers the Plans. Upon the adoption of the 2018 Incentive Award Plan, the Company ceased issuing awards under the 2016 Equity Incentive Plan.

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, 2024 and for the six-month period ended June 30, 2025 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, 2024	8,232,587	$ 12.57	5.35 years
Granted	222,050	$ 6.07	
Exercised	(6,563)	$ 5.29	
Forfeited	(387,769)	$ 15.55	
Outstanding at June 30, 2025	8,060,305	$ 12.26	5.11 years
Options exercisable at June 30, 2025	6,951,648	$ 12.79	4.65 years
Options vested and expected to vest at June 30, 2025	8,060,305	$ 12.26	5.11 years
Aggregate intrinsic value of options outstanding as of June 30, 2025	$ 2,004		
Aggregate intrinsic value of options exercisable as of June 30, 2025	$ 1,827		

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. Options granted to

directors when they join the board generally vest in 36 equal monthly installments following the date of grant, and 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 six-month periods ended June 30, 2025 and 2024 (in thousands):

	Three-Month Periods Ended June 30,	
	2025	2024
Research and development	$ 842	$ 1,579
General and administrative	500	1,024
Total share-based compensation	$ 1,342	$ 2,603

	Six-Month Periods Ended June 30,	
	2025	2024
Research and development	$ 1,783	$ 3,214
General and administrative	1,095	2,077
Total share-based compensation	$ 2,878	$ 5,291

The total fair value of options vested during the three-month periods ended June 30, 2025 and 2024 was $1.4 million and $3.2 million, respectively.

The total fair value of options vested during the six-month periods ended June 30, 2025 and 2024 was $3.4 million and $7.5 million, respectively.

The weighted-average grant date fair value of options granted during the six-month period ended June 30, 2025 and 2024 was $3.85 and $3.91 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:

	2025	2024
Risk-free interest rate	3.81 - 4.13%	4.04 - 4.17%
Expected volatility	67%	67%
Expected dividend yield	0%	0%
Expected term (in years)	6.01	3.6 - 6.1

As of June 30, 2025, the total compensation expense relating to unvested options granted that had not yet been recognized was $6.2 million, which is expected to be realized over a period of 3.9 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, 2024 and for the six-month period ended June 30, 2025 is as follows:

	Number of Restricted Share Units	Weighted-Average Grant Date Fair Value
Outstanding at December 31, 2024	4,252,250	$ 8.91
Granted	3,174,500	$ 6.17
Vested	(1,162,500)	$ 11.42
Forfeited	(12,000)	$ 6.16
Outstanding at June 30, 2025	6,252,250	$ 7.06

RSUs granted 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. 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. During the three and six months ended June 30, 2025, 225,000 RSUs granted to Directors vested and payment of shares was deferred. As of June 30, 2025, there were 497,500 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.

The Company recorded the following share-based compensation expense in connection with the RSUs for the three-month and six-month periods ended June 30, 2025 and 2024 (in thousands):

	Three-Month Periods Ended June 30,	
	2025	2024
Research and development	$ 1,249	$ 1,253
General and administrative	3,073	1,954
Total share-based compensation	$ 4,322	$ 3,207

	Six-Month Periods Ended June 30,	
	2025	2024
Research and development	$ 2,356	$ 2,493
General and administrative	5,317	4,986
Total share-based compensation	$ 7,673	$ 7,479

As of June 30, 2025, the total compensation expense relating to unvested RSUs granted that had not yet been recognized was $33.7 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 six months ended June 30, 2025 and 2024, the Company withheld 405,459 and 348,652 ordinary shares with a total value of approximately $2.7 million and $2.2 million, respectively. These amounts are presented as a cash outflow from financing activities in the accompanying condensed consolidated statement of cash flows.

During the three-month and six-month periods ended June 30, 2025 and 2024, the Company recognized total share-based compensation expense in the accompanying condensed consolidated statements of operations and comprehensive loss as follows (in thousands):

	Three-Month Periods Ended June 30,	
	2025	2024
Research and development	$ 2,091	$ 2,832
General and administrative	3,573	2,978
Total share-based compensation	$ 5,664	$ 5,810

	Six-Month Periods Ended June 30,	
	2025	2024
Research and development	$ 4,139	$ 5,707
General and administrative	6,412	7,063
Total share-based compensation	$ 10,551	$ 12,770

The Company does not expect to realize any tax benefits from its share option activity or the recognition of share-based compensation expense because the Company currently has net operating losses and has a full valuation allowance against its deferred tax assets. Accordingly, no amounts related to excess tax benefits have been reported in cash flows from operations or cash flows from financing activities for the six-month periods ended June 30, 2025 and 2024.

6. 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 six-month period ended June 30, 2025, the Company raised gross proceeds of $9.9 million through the sale of 1,510,300 ordinary shares 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 an additional $81.8 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.

7. Income Taxes

The Company did not record a provision for income taxes for the three-month and six-month periods ended June 30, 2025 and 2024, as the Company has generated losses for all periods.

The Company periodically evaluates the realizability of its deferred tax assets based on all available evidence, both positive and negative. The realization of deferred tax assets is dependent on the Company's ability to generate sufficient future taxable income during periods prior to the expiration of tax attributes to fully utilize these assets. The Company weighed both positive and negative evidence and determined that there is a continued need for a full valuation allowance on its deferred tax assets (after consideration of the reversal of the deferred tax liabilities for the ROU assets and fixed assets) in the United States, United Kingdom, Ireland and Netherlands as of June 30, 2025. Should the Company determine that it would be able to realize its remaining deferred tax assets in the foreseeable future, an adjustment to its remaining deferred tax assets would cause a material increase to income in the period such determination is made.

8. Related-Party Transactions

Asset Purchase and Related Agreements with Johnson & Johnson Innovative Medicine

On December 20, 2023, the Company entered into the Asset Purchase Agreement with Johnson & Johnson Innovative Medicine pursuant to which the Company sold and assigned to Johnson & Johnson Innovative Medicine, and Johnson & Johnson Innovative Medicine purchased and assumed, the UCLB RPGR 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. Simultaneously, the Company and Johnson & Johnson Innovative Medicine also entered into a Supply Agreement pursuant to which the Company agreed to manufacture and supply the RPGR Product for Johnson & Johnson Innovative Medicine. Under the Supply Agreement, MeiraGTx UK II, together with its affiliates, will manufacture commercial supply of the RPGR Product for Johnson & Johnson Innovative Medicine for an initial term of four years, with Johnson & Johnson Innovative Medicine having an option to extend the Supply Agreement for a fifth year upon written notification. Johnson & Johnson Innovative Medicine may terminate the Supply Agreement for convenience upon 90 days' written notice with payment of a termination fee. Under the Asset Purchase Agreement, Johnson & Johnson Innovative Medicine paid the Company a non-refundable upfront fee of $65.0 million in December 2023 and the Company is eligible to receive fees from commercial supply of the RPGR Product and in addition, milestones of up to $350.0 million, as follows: (i) a milestone payment of $50.0 million in connection with the achievement of the initiation of the extension study for the Phase 3 LUMEOS clinical trial for the RPGR Product; (ii) $10.0 million upon completion of certain specified development services for the drug substance for the RPGR Product; (iii) $5.0 million upon completion of certain specified development services for the drug product for the RPGR Product; (iv) $175.0 million upon the first commercial sale of an RPGR Product in the United States; (v) $75.0 million upon the first commercial sale of an RPGR Product in at least one of the United Kingdom, France, Germany, Spain and Italy; (vi) $25.0 million upon completion of the transfer of certain manufacturing technology for drug substance and drug product from the Company to Johnson & Johnson Innovative Medicine; and (vii) $10.0 million upon regulatory approval of a Johnson & Johnson Innovative Medicine-selected manufacturing facility in each of the United States and European Union for commercial manufacture of the RPGR Product. To date, the Company received $60.0 million in milestone payments from Johnson & Johnson Innovative Medicine. Johnson & Johnson Innovative Medicine is also responsible for any royalty or milestone amounts that become payable on the RPGR Product under the UCLB RPGR License Agreement.

Revenue Recognition under the Asset Purchase and Related Agreements

The agreements entered into in December 2023 were executed at the same time and were negotiated with a single commercial objective; therefore, the contracts were combined and accounted for as a single contract. These agreements were accounted for as a termination of the Collaboration Agreement and the creation of a new contract where the transaction price includes the remaining deferred revenue – related party from the terminated agreement of $30.6 million, the fixed upfront payment of $65.0 million under the Asset Purchase Agreement, and an aggregate of $1.8 million estimated variable consideration for transition services, offset by a credit of $5.1 million for pre-funded inventory, totaling $92.3 million. The transaction price was allocated to four performance obligations on a relative SSP basis, subject to certain exceptions for discounts and variable consideration. As the SSPs are not directly observable for any of the distinct goods and services, the SSPs were estimated based on a valuation. The total transaction price of $92.3 million was allocated to the performance obligations with respect to SSPs as follows: process performance qualification ("PPQ") services in the amount of $2.9 million net of future billings, material rights representing the commercial supply of RPGR Product and an in-substance contract renewal option in the amount of $6.9 million, manufacturing technology transfer in the amount of $28.7 million, and the sale of nonfinancial assets representing the sale and transfer of all the Company's right, title, and interest in the intellectual property related to the RPGR Product and the assignment of the UCLB RPGR License Agreement to Johnson & Johnson Innovative Medicine in the amount of $53.8 million.

Since the commencement of the Asset Purchase Agreement and related agreements, the Company received a $50.0 million milestone payment in connection with the achievement of the initiation of the extension study for the Phase 3 LUMEOS clinical trial for the RPGR Product. The milestone payment was allocated to the four performance

obligations on the same basis noted above increasing the value of each performance obligation as follows: PPQ services in the amount of $1.6 million, material rights representing the commercial supply of RPGR Product and an in-substance contract renewal option in the amount of $3.8 million, manufacturing technology transfer in the amount of $15.6 million, and the sale of nonfinancial assets representing the sale and transfer of all the Company's right, title, and interest in the intellectual property related to the RPGR Product and the assignment of the UCLB RPGR License Agreement to Johnson & Johnson Innovative Medicine in the amount of $29.0 million. Additionally, the Company received a $10.0 million milestone payment in connection with the completion of certain specified development services for the drug substance for the RPGR Product which was allocated to the PPQ services performance obligation. The Company also entered into additional agreements to provide additional services under the Asset Purchase Agreement and related agreements amounting to an aggregate of $5.8 million which was included in the transaction price and allocated to each of the respective performance obligations. Furthermore, the Company recorded certain changes in estimates related to the valuation of its performance obligations.

The transaction price allocated to PPQ services will be recognized over time using an inputs method measure of progress. The transaction price allocated to the material right for the commercial supply of RPGR Product will be recorded as deferred revenue until Johnson & Johnson Innovative Medicine exercises its option to purchase supply and the Company transfers control of such supply to Johnson & Johnson Innovative Medicine. The transaction price allocated to the in-substance renewal option (material right) will be recorded as deferred revenue until Johnson & Johnson Innovative Medicine exercises the option and the Company transfers control of the underlying goods or services to Johnson & Johnson Innovative Medicine. The Company will account for the exercise of the in-substance renewal option (material right) as a continuation of the existing contract (i.e., a change in the transaction price). The transaction price allocated to the technology transfer will be recognized over time using an inputs method measure of progress. The Company will recognize a gain for the difference between the carrying amount of the nonfinancial assets and the consideration allocated to that unit of account when control of the nonfinancial assets transfers in accordance with ASC 610-20, *Other Income - Gains and Losses from the Derecognition of Nonfinancial Assets*.

During the six-month period ended June 30, 2024, the Company recognized a gain of $29.0 million related to the sale of nonfinancial assets which is included in other income in the condensed consolidated statements of operations and comprehensive loss. There was no gain on sale of nonfinancial assets recognized during the six-month period ended June 30, 2025.

As of June 30, 2025, the aggregate transaction price allocated to unsatisfied performance obligations was $66.0 million which the Company expects to recognize over an estimated period of approximately 2.5 years.

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

Deferred revenue at December 31, 2024	$	62,403
Other amounts collected or invoiced		599
Deferred revenue recognized as service revenue during the six-month period ended June 30, 2025		(2,298)
Effects of exchange rate changes		5,248
Deferred revenue at June 30, 2025	$	65,952

During the three-month periods ended June 30, 2025 and 2024, the Company recognized $1.6 million and $0.3 million, respectively, of deferred revenue – related party as service revenue in connection with PPQ services under the Asset Purchase Agreement and related agreements.

During the six-month periods ended June 30, 2025 and 2024, the Company recognized $2.3 million and $1.0 million, respectively, of deferred revenue – related party as service revenue in connection with PPQ services under the Asset Purchase Agreement and related agreements.

During the three-month period ended June 30, 2025, the Company recognized $3.7 million of service revenue, inclusive of the $1.6 million of deferred revenue – related party recognized as service revenue, based on cumulative progress of PPQ services under the Asset Purchase Agreement and related agreements. The Company recognized

$1.3 million during the three-month period ended June 30, 2024 related to transition services the Company provided to Johnson & Johnson Innovative Medicine which was recorded as an offset to research and development expense.

During the six-month period ended June 30, 2025, the Company recognized $5.6 million of service revenue, inclusive of the $2.3 million of deferred revenue – related party recognized as service revenue, based on cumulative progress of PPQ services under the Asset Purchase Agreement and related agreements. The Company recognized $1.6 million during the six-month period ended June 30, 2024 related to transition services the Company provided to Johnson & Johnson Innovative Medicine which was recorded as an offset to research and development expense.

Debt Financing

On August 2, 2022 the Company, as borrower, and Meira UK II and Meira 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. 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. Refer to the discussion in Note 10 for further information related to the accounting for the debt financing.

9. 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 3 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 $1.3 million and $1.4 million for the three-month periods ended June 30, 2025 and 2024, respectively.

Total rent expense under these leases was $2.5 million and $2.9 million for the six-month periods ended June 30, 2025 and 2024, respectively.

There were no leases recognized during the six-month periods ended June 30, 2025 and 2024.

During the three months ended March 31, 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 ROU asset and lease liability reflected in the accompanying condensed consolidated balance sheets. The components of lease cost for the three-month and six-month periods ended June 30, 2025 and 2024 are as follows (in thousands):

	Three-Month Periods Ended June 30,	
	2025	2024
Finance lease cost		
Amortization of right-of-use assets	$ 305	$ 281
Total finance lease cost	305	281
Operating lease cost	1,113	1,437
Short-term lease cost	100	41
Total lease cost	$ 1,518	$ 1,759

	Six-Month Periods Ended June 30,	
	2025	2024
Finance lease cost		
Amortization of right-of-use assets	$ 588	$ 560
Total finance lease cost	588	560
Operating lease cost	2,397	2,872
Short-term lease cost	195	87
Total lease cost	$ 3,180	$ 3,519

Amounts reported in the condensed consolidated balance sheets for leases where the Company is the lessee as of June 30, 2025 and December 31, 2024 were as follows (in thousands):

	June 30, 2025	December 31, 2024
Operating leases		
Right-of-use assets	$ 5,820	$ 10,576
Capitalized lease obligations	$ 6,696	$ 11,576
Finance leases		
Right-of-use assets	$ 24,154	$ 22,198
Weighted-average remaining lease term		
Operating leases	3.9 years	3.6 years
Finance leases	173.4 years	173.9 years
Weighted-average discount rate		
Operating leases	11.7 %	8.8 %
Finance leases	8.0 %	8.0 %

Other information related to leases for the three-month and six-month periods ended June 30, 2025 and 2024 are as follows (in thousands):

	Three-Month Periods Ended June 30,	
	2025	2024
Cash paid for amounts included in the measurement of lease liabilities		
Operating cash flows from operating leases	$ 1,256	$ 1,432

	Six-Month Periods Ended June 30,	
	2025	2024
Cash paid for amounts included in the measurement of lease liabilities		
Operating cash flows from operating leases	$ 2,457	$ 2,851

Future minimum lease payments under non-cancellable leases as of June 30, 2025 are as follows (in thousands):

	Operating Leases
2025	$ 1,511
2026	2,597
2027	1,638
2028	1,432
2029	610
Total undiscounted lease payments	$ 7,788
Less: Imputed interest	(1,092)
Total lease liabilities	$ 6,696

10. Debt Financing

On August 2, 2022 the Company, and the Subsidiary Guarantors, entered into the Financing Agreement with Perceptive. On December 19, 2022, the Financing Agreement was converted to a 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. The Company and the Subsidiary Guarantors entered into a Consent and Amendment with Perceptive on August 10, 2023 (the "First Consent and Amendment), and the Company and the Subsidiary Guarantors entered into a second Consent and Amendment with Perceptive on December 20, 2023 (the "Second Consent and Amendment").

The Notes Purchase Agreement provides for an initial $75.0 million notes issuance (the "Tranche 1 Notes"). Pursuant to the First Consent and Amendment, the Company was able to request in its sole discretion, and Perceptive agreed to subscribe to purchase upon such request, an additional $25.0 million notes issuance (the "Tranche 2 Notes") at any time before August 2, 2024 subject to the terms of the Notes Purchase Agreement. Previously, the Company's request for issuance of the Tranche 2 Notes was to be determined at Perceptive's sole discretion. The Notes Purchase Agreement matures on August 2, 2026 and is interest-only during the term. The Company has the option to redeem outstanding principal notes at any time along with an applicable early redemption fee. Under each of the First Consent and Amendment and the Second Consent and Amendment, the Notes Purchase Agreement was amended to increase the applicable early redemption fee. Outstanding amounts under the Notes Purchase Agreement bear interest at a fluctuating rate per annum equal to 10.00% plus the secured overnight financing rate administered by the Federal Reserve Bank of New York for a one-month tenor, subject to a 1.00% floor. The annual interest rate was 14.33% at June 30, 2025. As of June 30, 2025, the outstanding balance of the Tranche 1 Notes was $75.0 million plus accrued interest of $2.7 million. During the three-month periods ended June 30, 2025 and 2024, the Company recorded interest expense of $2.7 million and $2.9 million, respectively. During the six-month periods ended June 30, 2025 and 2024, the Company recorded interest expense of $5.4 million and $5.8 million, respectively.

The Company's obligations under the Notes Purchase Agreement are secured by the Company's London, UK and Shannon, Ireland manufacturing facilities, $3.0 million of the Company's cash and the bank accounts of the Subsidiary Guarantors, and the issued and outstanding equity interests of the Subsidiary Guarantors.

The Notes Purchase Agreement imposes certain covenants and restrictions on the Company and the Subsidiary Guarantors, including restrictions pertaining to: (i) the incurrence of additional indebtedness, (ii) limitations on liens, (iii) limitations on certain investments, (iv) making distributions, dividends and other payments, (v) mergers, consolidations and acquisitions, (vi) dispositions of assets, (vii) the Company's maintenance of at least $3.0 million in a U.S. bank account, (viii) transactions with affiliates, (ix) changes to governing documents, (x) changes to certain agreements and leases and (xi) changes in control; however, certain of these restrictions contain exceptions which allow the Company to license, sell and monetize assets in its AAV-hAQP1 program in development to treat radiation-induced xerostomia, its AAV-GAD program in development to treat Parkinson's disease and its gene regulation platform technologies. As of June 30, 2025, the Company is in compliance with all covenants.

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. The warrants are exercisable immediately and expire on August 2, 2027. The Company recorded a debt discount of $2.3 million for the allocated fair value of the warrants.

The Company also capitalized certain lender and legal costs associated with the Notes Purchase Agreement totaling $2.1 million, which were recorded as a discount to the loan. The aggregate discount of $4.4 million is being amortized to interest expense over the term of the Notes Purchase Agreement. The Company amortized $0.3 million of the discount to interest expense during each of the three-month periods ended June 30, 2025 and 2024. The Company amortized $0.6 million and $0.5 million of the discount to interest expense during the six-month periods ended June 30, 2025 and 2024, respectively.

11. Commitments and Contingencies

There were no new material commitments or contingencies entered into during the six-month period ended June 30, 2025.

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, 2024 (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 late-stage clinical programs, including Parkinson's disease, radiation-induced xerostomia and AIPL1-associated retinal dystrophy. Our clinical programs use targeted local delivery of small doses of genetic medicines to treat both inherited and more common conditions with severe unmet need. The successful development of the clinical pipeline is supported by our internal end-to-end manufacturing capabilities. We have two GMP viral vector production facilities, internal plasmid production for GMP, as well as an in-house Quality Control hub for stability and release, all fit for IND through commercial supply. In addition, we have developed a proprietary manufacturing platform with leading yield and quality aspects and commercial readiness. Our core capabilities in viral vector and capsid optimization allow increased potency, decreased dose and significantly reduced cost of goods for our genetic medicines. We have developed a transformative gene regulation platform using bespoke synthetic riboswitch technology invented in-house that allows for the precise, dose-responsive control of any transgene under the control of oral small molecules. We are focusing the riboswitch platform on *in vivo* delivery of biologic therapeutics such as the metabolic peptides GLP-1, GIP, glucagon, amylin, PYY and leptin via oral small molecules, as well as cell therapy for oncology and autoimmune diseases, and long-term intractable pain. We have developed unique comprehensive technology capabilities to apply genetic medicine to more 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 viral vector manufacturing facilities and our GMP plasmid and DNA 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, proceeds from the sales of our equity securities, debt financing and upfront and milestone payments in connection with the Collaboration, Option and License Agreement with Johnson & Johnson Innovative Medicine (formerly known as Janssen Pharmaceuticals, Inc.), dated as of January 30, 2019 (the "Collaboration Agreement"), which also provided us with research funding, and the Asset Purchase Agreement, dated as of December 20, 2023, we entered into with Johnson & Johnson Innovative Medicine (the "Asset Purchase Agreement") pursuant to which we sold and assigned to Johnson & Johnson Innovative Medicine, and Johnson & Johnson Innovative Medicine purchased and assumed, that certain License Agreement, dated February 5, 2019, by and between UCL Business Plc (now UCL Business Ltd.) ("UCLB"), on the one hand, and MeiraGTx UK II Limited and MeiraGTx Limited, on the other hand (the "UCLB RPGR License Agreement"), relating to the research, development, manufacture and exploitation of botaretigene sparoparvovec, or bota-vec (formerly referred to as AAV-RPGR), for the treatment of X-linked retinitis pigmentosa related to mutations in the retinitis pigmentosa GTPase regulator gene, or XLRP-RPGR (the "RPGR Product"), and other related assets as described in the Asset Purchase Agreement. Through June 30, 2025, we received gross proceeds of approximately $632.2 million from sales of our equity securities, gross proceeds of

approximately $75.0 million from issuance of debt, $130.0 million in upfront and milestone payments from the Collaboration Agreement with Johnson & Johnson Innovative Medicine, and $125.0 million from the Asset Purchase Agreement with Johnson & Johnson Innovative Medicine. As of June 30, 2025, we had cash, cash equivalents and restricted cash of $34.4 million, as well as $2.3 million in receivables due from Johnson & Johnson Innovative Medicine in connection with PPQ and transition services we provided to Johnson & Johnson Innovative Medicine.

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 losses for the three-month periods ended June 30, 2025 and 2024 were $38.8 million and $48.6 million, respectively. Our net losses for the six-month periods ended June 30, 2025 and 2024 were $78.8 million and $69.1 million, respectively. As of June 30, 2025, we had an accumulated deficit of $780.8 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 June 30, 2025 and 2024 were $48.5 million and $46.2 million, respectively. For the six-month periods ended June 30, 2025 and 2024, our total operating expenses were $92.0 million and $93.7 million, respectively. We expect to continue incurring costs associated with our clinical activities for AAV-hAQP1 for the treatment of radiation-induced xerostomia and xerostomia associated with Sjogren's syndrome, AAV-GAD for the treatment of Parkinson's disease, as well as costs associated with the delivery of services under the Asset Purchase Agreement and related agreements. We also incurred expenses during the six-month period ended June 30, 2025 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 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 – related party and tax incentive receivable at June 30, 2025, together with the $17.0 million deposit received from Hologen Limited during the third quarter 2025 and the remaining proceeds from the anticipated closing of the strategic collaboration with Hologen Limited as described below, we estimate that such funds will be sufficient to enable us to fund our operating expenses and capital expenditure requirements into 2027 and to repay our debt obligation of $75.0 million to Perceptive (due in August 2026). This estimate does not include the $285.0 million in milestones we are eligible to receive under the Asset Purchase Agreement upon first commercial sale of an RPGR Product in the United States and in at least one of the United Kingdom, France, Germany, Spain and Italy, for completion of the transfer of certain manufacturing technology to Johnson & Johnson Innovative Medicine and upon regulatory approval of a Johnson & Johnson Innovative Medicine-selected manufacturing facility in each of the United States and European Union, or EU, for commercial manufacture of the RPGR Product. 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.

Hologen Transactions

On March 9, 2025 (the "Hologen Signing Date"), we and our affiliates entered into a strategic collaboration with Hologen Limited, a non-cellular company limited by shares incorporated in Guernsey ("Hologen"), and its affiliates. 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, we and Hologen entered into the Framework Agreements (as defined below), pursuant to which we and our affiliates will receive from Hologen an upfront cash payment of $200 million (the "Upfront Payment") on the Hologen Closing Date (as defined below), and Hologen will provide additional funding of up to an additional $230 million as further described below. As part of the strategic collaboration, we also received 250,000 Class A shares of Hologen at a nominal price. Furthermore, we have the option to receive up to an additional 500,000 Class A shares of Hologen at a nominal price if certain funding obligations as described herein are not met by Hologen. During June 2025, Hologen made a $6.0 million payment as part of its commitment toward the Upfront Payment, and made an additional $17.0 million payment during the third quarter 2025.

The closing of the transactions contemplated by the Framework Agreements (the "Hologen Closing Date") is expected to occur in the third calendar quarter of 2025, subject to customary closing and funding conditions.

Neuro Framework Agreement

On the Hologen Signing Date, we, MeiraGTx Neuro UK Limited, a private company limited by shares incorporated in England and one of our wholly-owned subsidiaries ("MeiraGTx Neuro UK"), Hologen Neuro AI Limited, a non-cellular company limited by shares incorporated in Guernsey and an affiliate of Hologen ("Hologen Neuro"), and Hologen, entered into that certain Framework Agreement (the "Neuro Framework Agreement"), pursuant to which, on the Hologen Closing Date, we, MeiraGTx Neuro UK, MeiraGTx Neuro I, LLC, a Delaware limited liability company and one of our wholly-owned subsidiaries ("MeiraGTx Neuro US"), Hologen, Hologen Neuro and Hologen Neuro AI UK Limited, a private company limited by shares incorporated in England and an affiliate of Hologen ("Hologen Neuro UK"), shall enter into a Collaboration and License Agreement (the "Hologen Collaboration Agreement") for 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 Hologen Collaboration Agreement.

On the Hologen Closing Date, under the Hologen Collaboration Agreement, MeiraGTx Neuro US will receive the applicable portion of the Upfront Payment in consideration for granting to Hologen Neuro and Hologen Neuro UK, as of the Hologen Closing Date and subject to the license granted by Hologen Neuro 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. On the Hologen Closing Date, (a) MeiraGTx Neuro UK will receive Class A shares of Hologen Neuro representing a 30% ownership of the issued share capital of Hologen Neuro, in consideration for the provision of services to Hologen Neuro and Hologen Neuro UK as specified in the Hologen Collaboration Agreement, including services relating to the development of the Clinical Programs and the Delivery Device and certain other transition services, and (b) Hologen Guernsey will receive Class B shares of Hologen Neuro representing a 70% ownership of the issued share capital of Hologen Neuro, in consideration for paying the applicable portion of the Upfront Payment to MeiraGTx Neuro US, as well as a commitment to provide additional capital of up to $230 million to fund the development of the Clinical Programs and the Delivery Device. Additionally, Hologen will license to Hologen Neuro its proprietary multi-modal generative foundation models (LMMs), or large medicine models, pursuant to a license agreement mutually agreeable to the parties.

As of the Hologen Closing Date, Hologen Neuro shall be governed by a board of directors comprised of three representatives designated by Hologen and two representatives designated by MeiraGTx Neuro UK, and certain material business decisions (as further enumerated in the Neuro Framework Agreement) will require the approval of at least 70% of the directors then in office.

Following the Hologen Closing Date and in accordance with the terms of the Hologen Collaboration Agreement, the parties shall negotiate in good faith and enter into clinical and commercial supply agreements, pursuant to which MeiraGTx Neuro UK (directly, or through affiliates or subcontractors) shall manufacture and supply AAV-GAD, AAV-BDNF and other potential locally delivered genetic medicines to the central nervous system.

Manufacturing Framework Agreement

On the Hologen Signing Date, MeiraGTx Manufacturing Limited, a private company limited by shares incorporated in England and one of our wholly-owned subsidiaries ("MeiraGTx Manufacturing"), MeiraGTx Limited, a private company limited by shares incorporated in England and one of our wholly-owned subsidiaries ("MeiraGTx Limited"), and Hologen, entered into that certain Framework Agreement (the "Manufacturing Framework Agreement" and, together with the Neuro Framework Agreement, the "Framework Agreements"), pursuant to which, on the Hologen Closing Date and in exchange for the applicable portion of the Upfront Payment, Hologen will acquire a minority interest in MeiraGTx Manufacturing, an entity that will comprise our flexible and scalable end-to-end genetic medicines manufacturing business. Hologen will also contribute a portion of the annual funding to MeiraGTx Manufacturing.

As of the Hologen Closing Date, MeiraGTx Manufacturing shall be governed by a board of directors comprised of three representatives designated by MeiraGTx Limited and two representatives designated by Hologen, and certain material business decisions (as further enumerated in the Manufacturing Framework Agreement) will require the approval of at least 70% of the directors then in office.

For a period of twelve months following the Hologen Closing Date, Hologen has an exclusive, irrevocable option to purchase additional shares in MeiraGTx Manufacturing at a specified price, such that following exercise of such option, Hologen shall own 40% of the issued share capital of MeiraGTx Manufacturing in the aggregate. In the event that Hologen does not exercise its option, we have an exclusive, irrevocable option to purchase all of the shares of MeiraGTx Manufacturing held by Hologen for the same price that Hologen paid for such shares. Such option shall be exercisable anytime beginning on the third anniversary of the Hologen Closing Date and ending three years thereafter.

Acquisition of Smart Immune Assets

In July 2025, our new, wholly-owned French subsidiary MeiraGTx Cell Therapies acquired through a French insolvency proceeding certain assets and operations 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. Following a public tender process, the Paris court of economic activities chose our offer to acquire a majority of Smart Immune's assets and operations, including twenty of its employees. We paid a purchase price of €250,000 plus a €100,000 transfer fee under a license agreement. As a result of the acquisition, we intend to advance the development of, among other things, off-the-shelf, allogenic ProTcell-derived CAR-T therapies that incorporate our riboswitch technology platform.

Recent Development Highlights and Anticipated Milestones

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

- In December 2024, we were granted RMAT designation by the FDA for AAV2-hAQP1 for the treatment of Grade 2/3 RIX.
- Following FDA interactions through the RMAT meeting process, we have aligned with the Agency on both the CMC and clinical requirements for the ongoing Phase 2 AQUAx2 randomized, double-blind, placebo-controlled study to support a potential BLA.
- The use of a single Patient Reported Outcome (PRO) as primary endpoint, the 12-month timeframe for the primary outcome measure, the pooling of placebo arms, and the statistical analyses are aligned with the FDA.
- The Phase 2 AQUAx2 (NCT05926765) randomized, double-blind, placebo-controlled study is currently enrolling the final high dose cohorts at multiple sites in the US, Canada and the U.K. with the target for completion of enrollment in the fourth quarter of 2025, and the potential for pivotal data read out late 2026.
- Plans for process performance qualification (PPQ) for AAV-hAQP1 manufactured in-house at MeiraGTx to support the BLA filing are underway following guidance and alignment with the FDA.

Additional indications for AAV2-hAQP1

- Pre-clinical data supports the use of AAV2-hAQP1 in xerostomia in Sjogren's disease.
- Additionally, this same AAV2-hAQP1 treatment has the potential to address xerostomia resulting from the use of PSMA radioligand treatments, as well as prophylaxis for xerostomia caused by this class of treatment.
- Importantly, manufacturing of AAV2-hAQP1 for all additional indications will be in-house at MeiraGTx and will be the same potentially commercially approved manufacturing process as used for AAV2-hAQP1 in the current pivotal RIX study.

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

- On May 8th, 2025, the FDA granted Regenerative Medicine Advanced Therapy (RMAT) designation to AAV-GAD for the treatment of Parkinson's disease not adequately controlled with anti-Parkinsonian medications.
- This RMAT was awarded following the presentation to the FDA of positive data from 3 clinical studies demonstrating the benefit of AAV-GAD when administered in a one-time stereotactic infusion to the subthalamic nucleus in the brain, Phase 1 dose escalation study (n=14), double blind sham-surgery controlled Phase 2 study (n=45) and double blind sham-surgery controlled Phase 1/2 clinical bridging study (n=14).
- In July 2025 the FDA completed a Good Clinical Practice (GCP) inspection of the recent AAV-GAD double blind randomized sham-surgery controlled bridging study resulting in a clean inspection with zero observations and no Form 483.
- We and our partner Hologen are currently in discussion with the FDA around use of AI driven analysis of imaging data from the proposed double-blind, sham-surgery controlled Phase 3 study with the potential to support a disease modification claim on the label for AAV-GAD for the treatment of Parkinson's disease.
- We plan to initiate this Phase 3 study of AAV-GAD in 2025 and will continue to work with the FDA to expedite the development of the program supported by our end-to-end in-house manufacturing capabilities.

Strategic Collaboration with Hologen AI:

- We and Hologen received clearances and approvals under the foreign direct investment laws (FDI) of the United Kingdom in the second quarter of 2025.
- We have received $23 million of the $200 million in cash consideration due post-FDI clearance, with the remainder expected in the third quarter of 2025, and we will be granted an additional 250,000 Class A shares of Hologen.
- We and Hologen are forming a joint venture, Hologen Neuro AI Ltd, with additional committed funding from Hologen of up to $230 million into the joint venture to finance the development of the AAV-GAD program in Parkinson's disease to commercialization, as well as other locally-delivered therapies to the CNS.
- The joint venture, Hologen Neuro AI Ltd, will use Hologen's proprietary multi-modal generative foundation models (LMMs).
- We will hold a 30% ownership in the joint venture and lead all clinical development and manufacturing.
- Hologen Neuro AI Ltd will enter into both clinical and commercial manufacturing supply agreements with us for exclusive manufacturing of AAV-GAD and other locally-delivered genetic medicines targeting the CNS.
- Hologen will own a minority stake in our manufacturing subsidiary and will contribute a portion of the annual funding and deploy Hologen's world leading generative AI capabilities to further accelerate the optimization of our proprietary manufacturing capabilities.
- As part of the Hologen collaboration we are moving forward with a new program for treatment of severe chronic neuropathic pain using the local delivery of an undisclosed vector. This includes trigeminal neuralgia, one of the most severe forms of pain and intractable to treatment. This program is expected to enter the clinic in 2025 with material manufactured in house at MeiraGTx.

RMAT Designation:

The requirements for receiving an RMAT designation include that the drug candidate is an advanced regenerative medicine, in this case a gene therapy; that the therapy is targeting a serious condition, in this case, Parkinson's disease; and that the applicant has presented clinical evidence demonstrating that the drug candidate has the potential to address an unmet need in the serious condition. The RMAT requirement for clinical data supporting a benefit in an unmet need is a high hurdle, with less than half of all RMAT designation applications granted.

RMAT designation includes the benefits of Fast Track and Breakthrough Therapy designations with rolling review and potential Priority Review of a product's BLA. RMAT designation also allows for increased interaction with the FDA and immediate multidisciplinary comprehensive discussions of the ongoing product development program, clinical trials and plans for expediting the manufacturing development strategy, both clinical and CMC.

AAV-AIPL1 for LCA4 Caused by Mutations in the AIPL1 Gene:

- In February 2025, we announced that data demonstrating the efficacy of rAAV8.*hRKp.AIPL1* for the treatment of LCA4 were published in *The Lancet* in a paper titled, "*Gene therapy in children with AIPL1-associated severe retinal dystrophy: an open-label, first-in-human interventional study*"; the Company held a webcast to review the data, and a replay of the webcast can be accessed here.
- Meaningful responses were observed in 11 out of 11 LCA4 children treated to date with AAV-AIPL1, with all children treated who were blind at birth now able to see following treatment with AAV-AIPL1.
- Following discussions with both the MHRA and FDA, based on the clinical data from the 11 treated children, and feedback from both agencies on a potentially expedited CMC PPQ package, we are preparing the submission of an MAA in the UK and a BLA in the US for AAV-AIPL1, a treatment for LCA4.
- AAV-AIPL1 for the treatment of LCA4 has orphan drug designation in the US and orphan designation in the European Union, and the Offices of Orphan Products Development and Pediatric Therapeutics of the FDA have granted Rare Pediatric Disease Designation (RPDD) to AAV-AIPL1-for the treatment of LCA4 retinal dystrophy.

Botaretigene Sparoparvovec for the Treatment of X-linked Retinitis Pigmentosa (XLRP):

- Data from the Phase 3 LUMEOS trial of botaretigene sparoparvovec (bota-vec) for the treatment of X-linked retinitis pigmentosa was presented by Dr. Michael Clark, the primary clinical lead on the study from Johnson &

Johnson Innovative Medicine, at the Foundation Fighting Blindness 2025 Retinal Therapeutics Innovation Summit on May 2nd, 2025.

- Following the release of the compelling Phase 3 data at their summit, the Foundation Fighting Blindness issued a public letter to Johnson & Johnson Innovative Medicine strongly supporting the filing and ultimate approval of this treatment for XLRP and stating that it had a remarkable benefit for many of the patients treated.
- The FDA has granted Fast Track and orphan drug designations to bota-vec and the regulatory authorities in the EU have granted Priority Medicines, or PRIME, advanced therapy medicinal product, or ATMP, and orphan drug designations to bota-vec. Johnson & Johnson Innovative Medicine is the sponsor of this program.
- We is eligible to receive up to $285 million upon the first commercial sales of bota-vec in the US and EU and manufacturing tech transfer.
- We also entered into a commercial supply agreement with Johnson & Johnson Innovative Medicine for bota-vec manufacturing, which we anticipate will generate additional revenue during the product launch. As part of this commercial supply agreement, we have now completed PPQ to potentially support CMC sections of global regulatory filings.

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

- We continue to progress our riboswitch technology platform in multiple potential indications, with an initial focus on obesity and metabolic disease, neuropathic pain and CAR-T.
- We have generated compelling preclinical data with metabolic peptides and hormones, including incretins, myokines and leptin, which indicates greater efficacy on weight loss as well as a positive impact on fat to muscle ratio and improvement in post prandial glucose control which is significantly greater than the same long acting peptide combinations.
- Pre-clinical data from Riboswitch delivered leptin is particularly compelling and likely to be the first IND using our riboswitch small molecule platform.
- We are in dialogue with regulatory agencies and intend to be ready to initiate first-in-human studies using the riboswitch platform in 2025.
- To complement our RiboCAR platform, we have acquired ProTcell technology via the acquisition of certain assets and operations of Smart Immune, which allows T-cell progenitors to be generated outside the body. Along with our RiboCAR, this technology provides a unique potential for allogeneic high performance RiboCAR-T. ProTcell technology has shown proof of concept in 20 patients treated in 3 clinical studies. Pre-clinical studies of ProT+ RiboCAR are ongoing.

Manufacturing:

United Kingdom (MeiraGTx UK II Ltd.)

Our UK manufacturing facility holds two authorizations issued by the MHRA:

- MIA(IMP) Licence (MIA(IMP) 45522) – Authorizing manufacturing, fill-finish, and QC testing of Investigational Medicinal Products (IMPs).
- Specials Licence (MS 45522) – Authorizing manufacturing, fill-finish, and QC testing of ‘Special’ medicinal products.

The UK facility was inspected in May 2024, and the licences were successfully renewed. The outcome of this inspection confirmed that the site was found to be in compliance with GMP requirements for Investigational Medicinal Products (IMPs) and was operating at the required compliance level to support an application for a commercial MIA licence.

Ireland (MeiraGTx Ireland DAC)

Our Shannon facility holds two authorizations issued by Ireland’s Health Products Regulatory Authority (HPRA):

- MIA Licence (M1316) – Authorizing QC testing of commercial products (awarded June 2023).

- MIA(IMP) Licence (IMP13221) – Authorizing QC testing of Investigational Medicinal Products (IMPs) (awarded September 2023/QC and 2025/MFG).

The QC laboratory is actively undertaking release and stability testing on PPQ batches.

The latest HPRA inspection in February 2025 was highly successful—both QC licenses were renewed, and viral vector manufacturing was added to the MIA(IMP) license. This means the Shannon site can manufacture material for use in clinical trials, a first-of-its-kind license for a gene therapy facility in Ireland.

Components of Our Results of Operations

Service Revenue – Related Party

Our service revenue consisted of the process performance qualification ("PPQ") services performed in connection with the Asset Purchase Agreement and related agreements.

Operating Expenses

Our operating expenses since inception have consisted primarily of general and administrative costs and research and development costs. Since 2024, we have incurred expenses classified as cost of service revenue – related party performed in connection with the Asset Purchase Agreement and related agreements.

Cost of Service Revenue – Related Party

Our cost of service revenue consisted of the PPQ services performed in connection with the 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 xerostomia associated with Sjogren's syndrome, as well as for AAV-GAD for the treatment of Parkinson's disease, although certain of these increases relating to AAV-GAD are expected to be offset by the funding provided by Hologen after the anticipated closing of the strategic collaboration we entered into with them. In addition, we 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 Gain (Loss)

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.

Interest Income

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

Interest Expense

Interest expense consists of interest expense and amortization of the debt discount in connection with the debt financing described in Note 10 to our condensed consolidated financial statements.

Gain on Sale of Nonfinancial Assets

The gain on sale of nonfinancial assets represents the value allocated to the nonfinancial assets sold and assigned to Johnson & Johnson Innovative Medicine including the UCLB RPGR License Agreement relating to the research, development, manufacture and exploitation of the RPGR Product, and other related assets pursuant to the Asset Purchase Agreement, net of carrying value.

Other Comprehensive Loss

Other comprehensive loss includes the following:

Foreign Currency Translation 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 on the condensed consolidated statements of operations and comprehensive loss.

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 service revenue, 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, 2024 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 June 30, 2025 and 2024

	2025	2024	Change
		(in thousands)	
Revenues:			
Service revenue - related party	$ 3,691	$ 282	$ 3,409
Total revenue	3,691	282	3,409
Operating expenses:			
Cost of service revenue - related party	2,676	—	2,676
General and administrative	12,313	11,257	1,056
Research and development	33,495	34,934	(1,439)
Total operating expenses	48,484	46,191	2,293
Loss from operations	(44,793)	(45,909)	1,116
Other non-operating income (expense)			
Foreign currency gain (loss)	8,624	(284)	8,908
Interest income	408	827	(419)
Interest expense	(3,034)	(3,254)	220
Net loss	$ (38,795)	$ (48,620)	$ 9,825

Service Revenue – Related Party

Service revenue was $3.7 million for the three months ended June 30, 2025, compared to $0.3 million for the three months ended June 30, 2024. The increase of $3.4 million was due to increased progress of PPQ services under the Asset Purchase Agreement and related agreements.

Cost of Service Revenue – Related Party

Cost of service revenue was $2.7 million for the three months ended June 30, 2025 due to progress of PPQ services under the Asset Purchase Agreement and related agreements. There was no cost of service revenue for the three months ended June 30, 2024.

General and Administrative Expenses

General and administrative expenses were $12.3 million for the three months ended June 30, 2025, compared to $11.3 million for the three months ended June 30, 2024. The increase of $1.0 million was primarily due to an increase of $0.6 million in legal and accounting fees, an increase of $0.6 million in share-based compensation and an increase of $0.1 million in payroll and payroll related costs. These increases were partially offset by a decrease of $0.3 million in consulting fees.

Research and Development Expenses

Research and development expenses for the three months ended June 30, 2025 and 2024 were as follows (in thousands):

	For the Three Months Ended June 30, 2025	2024	Change
Clinical Programs			
Botaretigene sparoparvovec	$ —	$ 816	$ (816)
AAV-hAQP1	2,754	4,617	(1,863)
AAV-CNGB3 / AAV-CNGA3	—	818	(818)
AAV-GAD	8,970	1,466	7,504
Other ocular diseases	20	—	20
Manufacturing	12,280	18,400	(6,120)
Preclinical Programs			
Gene regulation	3,241	3,101	140
Neurodegenerative diseases	298	361	(63)
Preclinical ocular diseases	1,162	583	579
Other research and development expenses	4,770	6,029	(1,259)
Gross research and development expenses	33,495	36,191	(2,696)
Johnson & Johnson Innovative Medicine reimbursement	—	(1,257)	1,257
Total research and development expenses	$ 33,495	$ 34,934	$(1,439)

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 for the three months ended June 30, 2025 were $33.5 million, compared to $34.9 million for the three months ended June 30, 2024. The decrease of $1.4 million was primarily due to a decrease of $6.1 million in manufacturing costs due to a reclassification of batch costs to the AAV-GAD program and reclassification of cost of service revenue due to progress of the PPQ services provided under the Asset Purchase Agreement and related agreements, a decrease of $1.9 million for the AAV-hAQP1 program resulting from no batch production during the current quarter, a decrease of $0.8 million in the AAV-CNBGB3 and AAV-CNBGA3 programs, a decrease of $0.8 million related to bota-vec as Johnson & Johnson Innovative Medicine is now primarily funding the expenses related to this program as a result of the Asset Purchase Agreement, a decrease of $0.1 million in neurodegenerative diseases research and a decrease of $1.2 million in other research and development expenses. These decreases were partially offset by an increase of $7.5 million in clinical trial expenses primarily due to an increase in costs associated with our AAV-GAD program, a reduction in reimbursements from Johnson & Johnson Innovative

Medicine of $1.3 million, an increase of $0.6 million in preclinical ocular diseases research costs, and an increase of $0.1 million in gene regulation research costs.

Foreign Currency Gain (Loss)

Foreign currency gain was $8.6 million for the three months ended June 30, 2025 compared to a loss of $0.3 million for the three months ended June 30, 2024. The change of $8.9 million was primarily due to the weakening of the U.S. dollar against the pound sterling and euro as it relates to the valuation of our intercompany payables and receivables.

Interest Income

Interest income was $0.4 million for the three months ended June 30, 2025 compared to $0.8 million for the three months ended June 30, 2024. The decrease of $0.4 million was due to lower interest rates and cash balances held in interest bearing accounts during 2025.

Interest Expense

Interest expense was $3.0 million for the three months ended June 30, 2025 compared to $3.3 million for the three months ended June 30, 2024. The decrease of $0.3 million was primarily due to a lower interest rate in connection with the debt financing described in Note 10 to our unaudited condensed consolidated financial statements included elsewhere in this Form 10-Q.

Comparison of Six Months Ended June 30, 2025 and 2024

	2025	2024	Change
		(in thousands)	
Revenues:			
Service revenue - related party	$ 5,617	$ 979	$ 4,638
Total revenue	5,617	979	4,638
Operating expenses:			
Cost of service revenue - related party	4,054	—	4,054
General and administrative	21,677	24,404	(2,727)
Research and development	66,275	69,256	(2,981)
Total operating expenses	92,006	93,660	(1,654)
Loss from operations	(86,389)	(92,681)	6,292
Other non-operating income (expense)			
Foreign currency gain (loss)	12,311	(819)	13,130
Interest income	1,379	1,924	(545)
Interest expense	(6,077)	(6,504)	427
Gain on sale of nonfinancial assets	—	29,018	(29,018)
Net loss	$ (78,776)	$ (69,062)	$ (9,714)

Service Revenue – Related Party

Service revenue was $5.6 million for the six months ended June 30, 2025 compared to $1.0 million for the six months ended June 30, 2024. The $4.6 million increase was due to progress of PPQ services under the Asset Purchase Agreement and related agreements.

Cost of Service Revenue – Related Party

Cost of service revenue was $4.1 million for the six months ended June 30, 2025, due to progress of PPQ services under the Asset Purchase Agreement and related agreements. There was no cost of service revenue for the six months ended June 30, 2024.

General and Administrative Expenses

General and administrative expenses were $21.7 million for the six months ended June 30, 2025, compared to $24.4 million for the six months ended June 30, 2024. The decrease of $2.7 million was primarily due to a $1.3 million gain on lease termination, a decrease of $0.7 million in share-based compensation, a decrease of $0.7 million in consulting fees, a decrease of $0.6 million in legal and accounting fees and a decrease of $0.2 million in other office related costs. These decreases were partially offset by an increase of $0.8 million in payroll and payroll-related costs.

Research and Development Expenses

Research and development expenses for the six months ended June 30, 2025 and 2024 were as follows (in thousands):

	For the Six Months Ended June 30,		
	2025	2024	Change
Clinical Programs			
Botaretigene sparoparvovec	$ —	$ 1,135	$ (1,135)
AAV-hAQP1	7,606	6,279	1,327
AAV-CNGB3 / AAV-CNGA3	—	(361)	361
AAV-GAD	10,430	3,636	6,794
Other ocular diseases	1,465	293	1,172
Manufacturing	29,640	40,397	(10,757)
Preclinical Programs			
Gene regulation	5,527	5,600	(73)
Neurodegenerative diseases	724	751	(27)
Preclinical ocular diseases	1,787	910	877
Other research and development expenses	9,096	12,525	(3,429)
Gross research and development expenses	66,275	71,165	(4,890)
Johnson & Johnson Innovative Medicine reimbursement	—	(1,909)	1,909
Research and development expenses	$ 66,275	$ 69,256	$ (2,981)

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 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 for the six months ended June 30, 2025 were $66.3 million, compared to $69.3 million for the six months ended June 30, 2024. The decrease of $3.0 million was primarily due to a decrease of $10.8 million in manufacturing costs primarily due to a reclassification of batch costs to the AAV-GAD program and reclassification of cost of service revenue due to progress of PPQ services provided under the Asset Purchase Agreement and related agreements, a decrease of $1.1 million related to the bota-vec program as Johnson & Johnson Innovative Medicine is now primarily funding the expenses related to this program as a result of the Asset Purchase Agreement, a decrease of $0.1 million related to gene regulation research costs, and a decrease of $3.4 million in other research and development costs. These decreases were partially offset by an increase of $6.8 million in clinical trial expenses primarily due to an increase in costs associated with our AAV-GAD program, an increase of $1.3 million for the AAV-hAQP1 program, an increase of $1.1 million in other ocular diseases, an increase of $0.9 million in preclinical ocular diseases, a reduction in a prior period credit of $0.4 million for the AAV-CNBGB3 and AAV-CNBGA3 programs, and a reduction in reimbursements from Johnson & Johnson Innovative Medicine of $1.9 million.

Foreign Currency Gain (Loss)

Foreign currency gain was $12.3 million for the six months ended June 30, 2025 compared to a loss of $0.8 million for the six months ended June 30, 2024. The change of $13.1 million was primarily due to the weakening of the U.S. dollar against the pound sterling and euro as it relates to the valuation of our intercompany payables and receivables.

Interest Income

Interest income was $1.4 million for the six months ended June 30, 2025 compared to $1.9 million for the six months ended June 30, 2024. The decrease of $0.5 million was due to lower interest rates and cash balances during 2025.

Interest Expense

Interest expense was $6.1 million for the six months ended June 30, 2025 compared to $6.5 million for the six months ended June 30, 2024. The decrease of $0.4 million was primarily due to a lower interest rate in connection with the debt financing described in Note 10 to our unaudited condensed consolidated financial statements included elsewhere in this Form 10-Q

Gain on Sale of Nonfinancial Assets

There was no gain on sale of nonfinancial assets during the six months ended June 30, 2025 compared to $29.0 for the six months ended June 30, 2024. This decrease was a result of the recognition of the $50.0 million milestone allocated to the nonfinancial assets sold and assigned to Johnson & Johnson Innovative Medicine including the UCLB RPGR License Agreement relating to the research, development, manufacture and exploitation of the RPGR Product, and other related assets described in the Asset Purchase Agreement in 2024.

Liquidity and Capital Resources

Since our inception, we have incurred significant operating losses. For the six months ended June 30, 2025, we used $80.8 million in cash flows from operations. We did not generate positive cash flows from operations during the period and 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, the issuance of debt and upfront and milestone payments from the Collaboration Agreement and Asset Purchase Agreement.

Based on our current cash, cash equivalents, accounts receivable – related party, and tax incentive receivable at June 30, 2025, together with the $17.0 million deposit received from Hologen during the third quarter 2025 and the remaining proceeds from the anticipated closing of the strategic collaboration with Hologen, we estimate that we will be able to fund our operating expenses and capital expenditure requirements into 2027 and to repay our debt obligation of $75.0 million to Perceptive (due in August 2026). This estimate does not include the $285.0 million in milestones we are eligible to receive under the Asset Purchase Agreement upon first commercial sale of an RPGR Product in the United States and in at least one of the United Kingdom, France, Germany, Spain and Italy, for completion of the transfer of certain manufacturing technology to Johnson & Johnson Innovative Medicine and upon regulatory approval of a Johnson & Johnson Innovative Medicine-selected manufacturing facility in each of the United States and European Union for commercial manufacture of the RPGR Product. 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 June 30, 2025, we had $34.4 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 Six Months Ended June 30,	
	2025	2024
	(in thousands)	
Net cash used in operating activities	$ (80,775)	$ (56,197)
Net cash (used in) provided by investing activities	(2,471)	26,011
Net cash provided by financing activities	7,163	333
Net decrease in cash, cash equivalents and restricted cash	$ (76,083)	$ (29,853)

Operating Activities

During the six months ended June 30, 2025, our cash used in operating activities of $80.8 million was primarily due to a net loss of $78.8 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 $3.5 million, which consisted primarily of $12.3 million of a foreign exchange gain, $10.6 million of share-based compensation, $6.2 million of depreciation and amortization, $1.4 million of gain on termination of lease liability, $0.1 million gain on disposal of equipment, furniture and fixtures, and $0.6 million of amortization of debt discount. Additionally, operating assets, consisting of accounts receivable – related party, prepaid expenses, tax incentive receivable, other assets and other current assets, decreased by $5.8 million and operating liabilities, consisting of accounts payable, accrued expenses and deferred revenue – related party, decreased by $11.4 million.

During the six months ended June 30, 2024, our cash used in operating activities of $56.2 million was primarily due to a net loss of $69.1 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 $8.0 million, which consisted primarily of $29.0 million of a gain on sale of nonfinancial assets, $12.8 million of share-based compensation, $6.5 million of depreciation and amortization, $0.8 million of a foreign currency loss, $0.5 million of amortization of debt discount and a $0.4 million loss on disposal of equipment, furniture and fixtures. Additionally, operating assets, consisting of accounts receivable – related party, prepaid expenses, tax incentive receivable, other assets and other current assets, decreased by $19.5 million and operating liabilities, consisting of accounts payable, accrued expenses and deferred revenue – related party, increased by $1.3 million.

Investing Activities

Net cash used in investing activities for the six months ended June 30, 2025 of $2.5 million consisted of purchases and disposals of property and equipment for our manufacturing, laboratory and process development facilities.

Net cash provided by investing activities for the six months ended June 30, 2024 of $26.0 million consisted of $29.0 million from proceeds from the sale of nonfinancial assets offset by $3.0 million of purchases of property and equipment for our manufacturing, laboratory and process development facilities.

Financing Activities

Net cash provided by financing activities was $7.2 million for the six months ended June 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 $2.8 million to cover tax withholding obligations upon the vesting of restricted share unit awards.

Net cash provided by financing activities was $0.3 million for the six months ended June 30, 2024, which consisted of $2.5 million of proceeds from an "at-the-market" offering of our ordinary shares, net of one-time costs relating to the preparation and filing of the shelf registration statement, accounting and legal fees, and negotiation of the sales agreement offset by the payment of $2.2 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, 2024 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 June 30, 2025, we did not hold any foreign currency forward contracts. With respect to our foreign currency exposures as of June 30, 2025, we estimate a 10% unfavorable movement in foreign currency exchange rates would have the effect of creating an additional foreign currency loss of approximately $2.2 million within other non-operating income (expense) for the six months ended June 30, 2025.

Interest Rate Risk

We are exposed to market risk as a result of changes in interest rates applicable to borrowings under our Notes Purchase Agreement. Borrowings under the Notes Purchase Agreement bear interest at a fluctuating rate per annum equal to 10.00% plus the secured overnight financing rate ("SOFR") administered by the Federal Reserve Bank of New York for a one-month tenor, subject to a 1.00% floor. See Note 10 to our unaudited condensed consolidated financial statements included elsewhere in this Form 10-Q. We may use interest rate cap derivatives, interest rate swaps or other interest rate hedging instruments to economically hedge and manage interest rate risk with respect to our variable floating rate debt. As of June 30, 2025, the annual interest rate was 14.33% and the outstanding balance of the term loan was $75.0 million. Assuming no change in the outstanding borrowings under the Notes Purchase Agreement, we estimate that a hypothetical 1% increase in the SOFR would increase our annual interest expense by approximately $0.8 million as of June 30, 2025.

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-15I 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 June 30, 2025 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. We were formed and began operations in 2015. We have never been profitable and do not expect to be profitable in the foreseeable future. We have incurred net losses since inception, including net losses of approximately $78.8 million and $69.1 million for the six months ended June 30, 2025, and 2024, respectively. As of June 30, 2025, we had an accumulated deficit of approximately $780.8 million. Since our inception, we have devoted substantially all of our resources to developing our technology platform, establishing our viral vector manufacturing facilities and plasmid and 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 large-scale, pivotal clinical trials, obtain marketing approval, 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 for the foreseeable 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 and research, preclinical and clinical activities for our riboswitch platform, although certain of these increases are expected to be offset by the funding provided by Hologen after the anticipated closing of the strategic collaboration we entered into with them. 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, 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.

There is no guarantee that we will receive in a timely fashion or at all the additional milestone payments contemplated under the Asset Purchase Agreement or the revenues associated with our manufacture of the commercial supply of the RPGR Product under the Supply Agreement.

On December 20, 2023, we and MeiraGTx UK II Limited entered into and consummated the Asset Purchase Agreement with Johnson & Johnson Innovative Medicine pursuant to which we sold and assigned to Johnson & Johnson Innovative Medicine, and Johnson & Johnson Innovative Medicine purchased and assumed, the UCLB RPGR 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. MeiraGTx UK II Limited and Johnson & Johnson Innovative Medicine also entered into a Supply Agreement on December 20, 2023 (the "Supply Agreement") pursuant to which MeiraGTx UK II Limited, together with its affiliates, will manufacture commercial supply of the RPGR Product for Johnson & Johnson Innovative Medicine for an initial term of four years, with Johnson & Johnson Innovative Medicine having an option to extend the Supply Agreement for a fifth year upon written notification to us.

Under the Asset Purchase Agreement, Johnson & Johnson Innovative Medicine paid us a non-refundable upfront cash purchase price of $65.0 million in December 2023. Additionally, pursuant to and subject to the terms and conditions set forth in the Asset Purchase Agreement, Johnson & Johnson Innovative Medicine agreed to pay us future contingent consideration of up to an aggregate of $350.0 million, as follows: (i) a milestone payment of $50.0 million in connection with the achievement of the initiation of the extension study for the Phase 3 LUMEOS clinical trial for the RPGR Product; (ii) $10.0 million upon completion of certain specified development services for the drug substance for the RPGR Product; (iii) $5.0 million upon completion of certain specified development services for the drug product for the RPGR Product; (iv) $175.0 million upon the first commercial sale of an RPGR Product in the United States; (v) $75.0 million upon the first commercial sale of an RPGR Product in at least one of the United Kingdom, France, Germany, Spain and Italy; (vi) $25.0 million upon completion of the transfer of certain manufacturing technology for drug substance and drug product from us to Johnson & Johnson Innovative Medicine; and (vii) $10.0 million upon regulatory approval of a Johnson & Johnson Innovative Medicine-selected manufacturing facility in each of the United States and European Union for commercial manufacture of the RPGR Product. To date, we received $60.0 million in milestone payments from Johnson & Johnson Innovative Medicine.

In connection with the sale and assignment of the UCLB RPGR License Agreement relating to the research, development, manufacture and exploitation of the RPGR Product to Johnson & Johnson Innovative Medicine, Johnson & Johnson Innovative Medicine has control and broad discretion over all aspects of the development and commercialization of the RPGR Product and we will have little, if any, influence over how such activities will be conducted. Johnson & Johnson Innovative Medicine will also be responsible for seeking regulatory approval and initiating the first commercial sale in the relevant jurisdictions of the RPGR Product, as well as obtaining regulatory approval of its manufacturing facilities in the relevant jurisdictions for the purposes of conducting commercial manufacture of the RPGR Product. These regulatory approvals and initiation of the first commercial sales in the relevant jurisdictions would entitle us to receive milestone payments up to an aggregate of $260.0 million. Our receipt of these milestones is dependent on Johnson & Johnson Innovative Medicine's ability to successfully develop and commercialize the RPGR Product and obtain the necessary regulatory approvals for its manufacturing facilities. If these regulatory approvals or commercial sales do not occur in a timely fashion or at all, then such milestone payments, and any revenues we may receive from manufacturing commercial supply of the RPGR Product, may be delayed or we may not receive such payments. Additionally, certain of these milestone based payments are payable upon our achievement of the specified development services, completion of the transfer of certain manufacturing technology to Johnson & Johnson Innovative Medicine and our ability to manufacture sufficient commercial supply of the RPGR Product in a timely fashion. In the event we are not successful in completing these activities in a timely fashion or at all, we will not receive the milestone payments associated with the relevant milestone under the Asset Purchase Agreement or receive revenue for commercial supply of the RPGR Product under the Supply Agreement. In each of these circumstances, our anticipated cash inflows from these activities would be reduced or eliminated, which would have an adverse effect on our revenue and financial position.

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 continue to expand our manufacturing and supply chain capabilities. This will require additional capital, which we may raise through equity offerings, debt financings, 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 June 30, 2025, our cash, cash equivalents and restricted cash were $34.4 million. In addition, we expect to receive $2.3 million from receivables from Johnson & Johnson Innovative Medicine during the third quarter of 2025 in connection with transition services we provided to Johnson & Johnson Innovative Medicine. Based on our cash, cash equivalents, accounts receivable – related party and tax incentive receivable at June 30, 2025, together with the $17.0 million deposit received from Hologen during the third quarter 2025 and the remaining proceeds from the anticipated closing of the strategic collaboration with Hologen, we estimate that such funds will be sufficient to enable us to fund our operating expenses and capital expenditure requirements into 2027 and to repay our debt obligation of $75.0 million to Perceptive (due in August 2026). This estimate does not include the $285.0 million in milestones we are eligible to receive under the Asset Purchase Agreement upon first commercial sale of an RPGR Product in the United States and in at least one of the United Kingdom, France, Germany, Spain and Italy, for completion of the transfer of certain manufacturing technology to Johnson & Johnson Innovative Medicine and upon regulatory approval of a Johnson & Johnson Innovative Medicine-selected manufacturing facility in each of the United States and European Union for commercial manufacture of the RPGR Product. 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 clinical development for our radiation-induced xerostomia product candidate, AAV-hAQP1, and for our product candidate for the treatment of Parkinson's disease, AAV-GAD;
- the progress, timing, costs and results of our ongoing clinical development for our AAV-AIPL1 gene therapy product candidate, the clinical development for our CNGB3 achromatopsia gene therapy product candidate, AAV-CNGB3, the clinical development for our CNGA3 achromatopsia gene therapy product candidate, AAV-CNGA3, and for the clinical development of our RPE65-associated retinal dystrophy product candidate, AAV-RPE65;
- the development of our product candidate for the treatment of ALS, AAV-UPF1, for 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 development of our potentially 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 extent to which we receive the milestone payments under the Asset Purchase Agreement with Johnson & Johnson Innovative Medicine;
- 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 or convertible debt securities 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. For example, in connection with entering into the Financing Agreement (as defined below), we issued warrants to Perceptive (as defined below), to purchase 400,000 ordinary shares at an exercise price of $15.00 per share and 300,000 ordinary shares at an exercise price of $20.00 per share. 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 August 2, 2022, we, as borrower, and our wholly-owned subsidiaries MeiraGTx UK II Limited and MeiraGTx Ireland DAC, as guarantors (the "Subsidiary Guarantors"), entered into a senior secured financing arrangement (the "Financing Agreement") by and among us, 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 (as converted, 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. We and the Subsidiary Guarantors entered into a Consent and Amendment with Perceptive on August 10, 2023 (the "First Consent and Amendment), and we and the Subsidiary Guarantors entered into a second Consent and Amendment with Perceptive on December 20, 2023 (the "Second Consent and Amendment"). The Notes Purchase Agreement provides for an initial $75.0 million notes issuance (the "Tranche 1 Notes"). Pursuant to the First Consent and Amendment, we were able to request in our sole discretion, and Perceptive agreed to subscribe to purchase upon such request, an additional $25.0 million notes issuance (the "Tranche 2 Notes", together with the Tranche 1 Notes, the "Notes") at any time before August 2, 2024, subject to the terms of the Notes Purchase Agreement. Previously, the Company's request for issuance of the Tranche 2 Notes was to be determined at Perceptive's sole discretion. Under each of the First Consent and Amendment and the Second Consent and Amendment, the Notes Purchase Agreement was also amended to increase the applicable early redemption fee. The Notes incur interest, subject to certain provisions therein, at a fluctuating rate per annum equal to 10.00% plus the secured overnight financing rate administered by the Federal Reserve Bank of New York for a one-month tenor, subject to a 1.00% floor. The Notes Purchase Agreement matures on August 2, 2026 and is interest-only during the term. The Notes Purchase Agreement also contains various restrictions and covenants, including, among other things, covenants regarding the incurrence of additional indebtedness, limitations on liens, limitations on certain investments, limitations on making distributions, dividends and other payments, mergers, consolidations and acquisitions, dispositions of assets, maintenance of at least $3.0 million in a U.S. bank account, transactions with affiliates, changes to governing documents, changes to certain agreements and leases and changes in control. Our obligations under the Notes Purchase Agreement are secured by our London, UK and Shannon, Ireland manufacturing facilities, $3.0 million of our cash and the bank accounts of the Subsidiary Guarantors, and the issued and outstanding equity interests of the Subsidiary Guarantors.

There can be no assurance that our cash and cash equivalents available under the Notes Purchase Agreement and under any future financings, together with any funds generated by our operations, will be sufficient to satisfy our debt payment obligations. Our inability to generate funds, obtain financing sufficient to satisfy our debt payment obligations or remain in compliance with the debt covenants may result in such obligations being accelerated by our lenders, which would likely have a material adverse effect on our business, financial condition and results of operations.

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. Our Notes Purchase Agreement provides that our breach or failure to satisfy certain covenants constitutes an event of default. Upon the occurrence of an event of default, in addition to an increase in the rate of interest on the Notes of 3% per annum, Perceptive could elect to declare all amounts outstanding thereunder to be immediately due and payable, proceed against the assets we provided as collateral, and, if such debt were accelerated, we may not have sufficient cash on hand or be able to sell sufficient collateral to repay it, which would have an immediate adverse effect on our business and operating results. This could potentially cause us to cease operations and result in a complete loss of your investment in our ordinary shares**.**

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. For example, in March 2025 we entered into a strategic collaboration with Hologen that is subject to customary closing and funding conditions that must be satisfied prior to completion of the transaction. It is possible that the transaction may not be completed or may not be completed as quickly as expected, which could have a material adverse effect on the price of our ordinary shares. In addition, any significant delay in consummating or a failure to consummate the 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 AAV-hAQP1, AAV-GAD, AAV-AIPL1 and our riboswitch gene regulation technology platform, as well as potentially AAV-CNGB3, AAV-CNGA3, AAV-RPE65, 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. 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 EU until we receive approval for an 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 a marketing authorization, or 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 completed any clinical development programs required for the approval of any of our product candidates. 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.

It is currently unclear to what extent the UK will seek to align its regulations with the EU. The UK regulatory framework in relation to clinical trials is derived from pre-existing EU legislation (as implemented into UK law, through secondary legislation). The extent to which the regulation of clinical trials in the UK may mirror the (EU) CTR in the long term is not yet certain. In December 2024, the UK government introduced a legislative proposal which, if implemented, could provide a more flexible regime to make it easier to conduct clinical trials in the UK, increase the transparency of clinical trials conducted in the UK and make clinical trials more patient centered. The legislation may not be approved or could be approved with amendment, and any adoption into UK law may not be until early 2026. Under the terms of the Protocol on Ireland and Northern Ireland, provisions of the (EU) CTR which relate to the manufacture and import of investigational medicinal products and auxiliary medicinal products currently apply in Northern Ireland. A decision by the UK not to closely align its regulations with the new approach adopted in the EU may have an effect on the cost of conducting clinical trials in the UK as opposed to other countries.

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. Additionally, major central bank policy actions may have a negative impact on our payment obligations under the Notes Purchase Agreement.

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 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 in the treatment of achromatopsia associated with defects in CNGB3 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. 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 AAV-CNGB3, AAV-CNGA3, AAV-RPE65, AAV-AIPL1, 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 disease 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. Under the FDA's regulations, the FDA will deny orphan drug exclusivity to a designated drug upon approval if the FDA has already approved another drug with the same principal molecular structural features, in the case of a biologic, for the same indication, unless the drug is demonstrated to be clinically superior to the previously approved drug. 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 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, for AAV-AIPL1 for the treatment of inherited retinal dystrophy due to defects in *AIPL1* gene 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 disease or condition 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 $100 million to $158 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. Under the current statutory provisions, the FDA may not award a rare pediatric disease priority review voucher to sponsors of marketing applications unless the drug has received rare pediatric disease designation as of December 20, 2024 and is approved by the FDA no later than September 30, 2026. Even though we received rare pediatric disease designation by the current statutory deadline of December 20, 2024 for all of these gene therapy product candidates except AAV8-RK-RetGC, which received such designation in January 2025, we may not receive a voucher for such gene therapy product candidates if we do not obtain approval by September 2026. It is possible that Congress may retroactively extend the date by which a rare pediatric disease-designated drug may be designated as such to be eligible for a priority review voucher, or extend the date by which a rare pediatric disease-designated drug must obtain approval in order to receive a priority review voucher, but even if such legislation is enacted, we may not obtain approval by that date, and even if we do, we may not obtain a priority review voucher.

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 our 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 our 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, as well as our obligations under the Supply Agreement with Johnson & Johnson Innovative Medicine if the RPGR Product is successfully commercialized, 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 instance, the EU pharmaceutical legislation is currently undergoing a complete review process, in the context of the Pharmaceutical Strategy for Europe initiative, launched by the European Commission in November 2020. The European Commission's proposal for revision of several legislative instruments related to medicinal products (potentially reducing the duration of regulatory data protection, revising the eligibility for expedited pathways, etc.) was published on April 26, 2023. The proposed revisions remain to be agreed and adopted by the European Parliament and European Council and the proposals may therefore be substantially revised before adoption, which is not expected before early 2026. The revisions may however have a significant impact on the pharmaceutical industry 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 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, following its relocation to Amsterdam and related reorganization (including staff changes), may also slow the time necessary for new product candidates to be reviewed and/or approved, which would adversely affect our business. For example, in recent years, the U.S. government has shut down several times and certain regulatory agencies, such as the FDA, have had to furlough critical FDA employees and stop critical activities. In addition, the current presidential administration has laid off thousands of federal health workers, including at the FDA, and the leadership of CBER has changed multiple times in recent months, 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 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 Medicaid drug rebate cap 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 beginning in 2026, 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 will first be effective in 2026, and the list of the subsequent 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.

Moreover, payment methodologies may 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. In addition, recently there has been heightened governmental scrutiny over the manner in which manufacturers set prices

for their marketed products, which has resulted in several U.S. Congressional inquiries and proposed and enacted federal legislation designed to, among other things, bring more transparency to drug pricing, reduce the cost of prescription drugs under Medicare, and review the relationship between pricing and manufacturer patient programs. 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, 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. We expect the existing legal complexity and uncertainty regarding international personal data transfers to continue. In particular, we expect the adequacy of the DPF as an approved GDPR transfer mechanism to be challenged and international transfers to the United States and to other jurisdictions more generally to continue to be subject to enhanced scrutiny by regulators. If, owing to the restriction or perceived restriction of personal data transfers, we are otherwise unable to transfer personal data between and among countries and regions in which we operate, it could affect the manner in which we provide our services, the geographical location or segregation of our relevant systems and operations, and could adversely affect our financial results.

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 affects 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 Applied Genetic Technologies Corporation, and 4D Molecular Therapeutics, Inc. There are a number of companies developing gene therapy products for neurodegenerative diseases, including Voyager Therapeutics, Inc., Brain Neurotherapy Bio, Inc., and Eli Lilly and Company. 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 FDA 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. We may pursue collaborative arrangements regarding the sale and marketing of AAV-hAQP1, AAV-GAD, our 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. 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 our 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, completed in early 2018, and our second, large scale GMP viral vector manufacturing facility and our first GMP plasmid and DNA production facility came online in 2022 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 have also agreed to manufacture commercial supply of the RPGR Product for Johnson & Johnson Innovative Medicine, if and when approved, under the Supply Agreement. If we fail to meet our obligations under the Supply Agreement, we may not be able to find a third-party manufacturer suitable to us or Johnson & Johnson Innovative Medicine to perform such manufacturing obligations, which could negatively impact our receipt of revenues under the Supply Agreement. While we now have our own plasmid manufacturing capabilities in our Shannon, Ireland facilities, we also rely on third-party manufacturers from time to time for the manufacture of plasmid used in the production of some 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 Collaboration Agreement with Johnson & Johnson Innovative Medicine for the development and commercialization of AAV-CNGB3, AAV-CNGA3 and bota-vec, which Collaboration Agreement was terminated in December 2023 in connection with our entering into the Asset Purchase Agreement with Johnson & Johnson Innovative Medicine. 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 entered into a strategic collaboration with Hologen in March 2025 as described above that is expected to close in the third calendar quarter of 2025 subject to customary closing and funding conditions. 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 we are therefore 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 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 unregistered trademarks or trade names. 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 Growth

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 June 30, 2025, 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 Stuart Naylor, Ph.D., our Chief Development Officer, as well as the other 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 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. 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 release 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 our shelf registration statement, including through our "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 six month period ended June 30, 2025, the Company raised gross proceeds of $9.9 million through the sale of 1,510,300 ordinary shares pursuant to an "at-the-market" equity offering program. Whether we choose 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 our ordinary shares relative to other sources of capital. The issuance from time to time of ordinary shares through our "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 June 30, 2025, 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 55.2% of our outstanding ordinary shares. In addition, in connection with entering into the Financing

Agreement, we issued to an affiliate of Perceptive Advisors, LLC, our largest shareholder that employs a director serving on our board, 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, 2024, 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, 2024 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, 2024, we had federal and state NOL carryforwards in the United States of $74.3 million and $19.3 million, respectively, and cumulative carryforward tax losses in the UK of $203.7 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, 2024, cumulative carryforward tax losses in Ireland were $78.3 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, 2024, we also had orphan drug and research and development credits in the U.S. in the amount of $22.5 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 $1.9 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 August 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 August 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 August 2024, we conducted an equity financing by selling an aggregate of 12.75 million ordinary shares at a price of $4.00 per share for gross proceeds of $51.0 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 expenses. 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 is an increasing focus from the SEC, foreign regulators, stock exchanges, certain investors and other stakeholders concerning corporate responsibility, specifically related to environmental, social and governance factors. The SEC has adopted rules regarding new climate-related disclosure, which have been stayed by the SEC pending the outcome of pending litigation challenging the new rules. 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 Notes Purchase Agreement prohibits us from paying 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, 2024 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.

During the three months ended June 30, 2025, none of our directors or "officers" (as defined in Rule 16a-1(f) under the Exchange Act) adopted, modified or terminated a "Rule 10b5-1 trading arrangement" and/or "non-Rule 10b5-1 trading arrangement," as each term is defined in Item 408(a) of Regulation S-K.

Item 6. Exhibits.

Exhibit Number	Description	Form	File No.	Exhibit	Filing Date	Filed/Furnished Herewith
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.

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: August 14, 2025

By: /s/ Alexandria Forbes

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

Date: August 14, 2025

By: /s/ Richard Giroux

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

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 June 30, 2025 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: August 14, 2025

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 June 30, 2025 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: August 14, 2025

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 June 30, 2025 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: August 14, 2025

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 June 30, 2025 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: August 14, 2025

By: /s/ Richard Giroux

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