MeiraGTx Reports First Quarter 2026 Financial and Operational Results

May 14, 2026

- *Received FDA Breakthrough Therapy Designation for AAV2-hAQP1*
- *Reported positive three-year data from the Phase 1 AQUAx study of AAV2-hAQP1 for the treatment of grade 2/3 late radiation-induced xerostomia*
- *Entered into an asset purchase agreement with Johnson & Johnson* (J&J) to acquire all interests in botaretigene sparoparvovec (bota-vec) for the treatment of X-linked retinitis pigmentosa (XLRP)*
- *Strengthened balance sheet with $100 million financing*

LONDON and NEW YORK, May 14, 2026 (GLOBE NEWSWIRE) -- MeiraGTx Holdings plc (Nasdaq: MGTX), a vertically integrated, clinical stage genetic medicines company, today announced financial and operational results for the first quarter ended March 31, 2026, and provided a corporate update.

“Our achievements in the first few months of 2026 have materially strengthened MeiraGTx – we are now in a position to file for potential approval and launch two wholly-owned therapies in the next 2 years,” said Alexandria Forbes, Ph.D., president and chief executive officer of MeiraGTx. “The compelling three-year durability data from our AAV2-hAQP1 Phase 1 study continue to demonstrate disease-modifying benefit following a simple one-time treatment of patients with moderate to severe persistent radiation-induced xerostomia, an otherwise debilitating life-long condition. The recent receipt of Breakthrough Therapy Designation for this program reinforces the strength of the data and the potential for an expedited development and regulatory pathway.”

Dr. Forbes continued, “We are very excited to have re-acquired bota-vec for the treatment of X-linked retinitis pigmentosa (XLRP). This is a highly strategic addition to our pipeline, given our long-term experience developing this drug while partnered with J&J, our deep expertise in ophthalmology, and our long-standing relationships with the inherited retinal disease patient community and KOL networks globally. Data from the Phase 3 LUMEOS study of bota-vec highlight the potential of this therapy to improve vision and significantly change the lives of those suffering with this otherwise inexorably degenerative disease. We are now working expeditiously to complete regulatory submissions in the U.S., EU, UK and Japan.”

“To that end, I am extremely pleased to announce that Penny Fleck has joined MeiraGTx as Chief Development Officer,” said Dr. Forbes. “Penny brings tremendous experience from her 20+ years at J&J and Takeda leading development of many assets, including multiple global regulatory approvals. Importantly, while at J&J as Global Head of Specialty Ophthalmology, Penny worked closely with MeiraGTx on the development of bota-vec, from the licensing of the drug by J&J through Phase 3. Her extensive experience and broad expertise across drug development will help us achieve the potential approvals of bota-vec and AAV2-hAQP1, as well as progress our early stage programs such as Ribo-Leptin into the clinic and through development. Stuart Naylor, Ph.D. will be taking on a new role with the Company as Chief Scientific Officer, Ophthalmology. Stuart is a founder of MeiraGTx and led the incorporation of the UCL ophthalmology assets into the Company, including AAV-RPGR. He has experience with this therapy from pre-clinical through Phase 3 and he will be focusing his efforts on obtaining global regulatory approval of bota-vec as well as advancing our ophthalmology product candidates at all stages of development.”

*Janssen Pharmaceuticals, Inc., a Johnson & Johnson company

First Quarter 2026 Highlights

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

- MeiraGTx entered into an asset purchase agreement with Johnson & Johnson in April 2026 to acquire full rights to bota-vec, a late-stage therapy for the treatment of X-linked retinitis pigmentosa (XLRP).
- MeiraGTx paid J&J a one-time $25 million upfront cash consideration, and J&J is eligible to receive a one-time regulatory and commercial milestone tied to U.S. approval and U.S. sales performance of bota-vec, as well as a mid-teens royalty on global net sales starting in mid-2029.
- The Company plans to rapidly advance the program toward global regulatory filings in the U.S., Europe, UK and Japan, leveraging its prior involvement in the program’s development and its established manufacturing readiness.

Bota-vec for the Treatment of X-linked Retinitis Pigmentosa (XLRP):

- XLRP is a rare inherited retinal disease with early onset and progressive degeneration to complete blindness in the third decade of life. There are currently no treatment options.
- There are >20,000 XLRP-RPGR patients in the U.S. and EU.
- The Phase 3 LUMEOS study was a global randomized study (n=95). All patients were treated bilaterally.

- Data from the Phase 3 LUMEOS trial of bota-vec for the treatment of XLRP was presented at the Foundation Fighting Blindness 2025 Retinal Therapeutics Innovation Summit.
- Safety profile of bota-vec was as expected and manageable, with no new safety signals in the Phase 3 study with improved inflammatory profile compared to the Phase 1/2 study.
- As the commercial manufacturer of bota-vec, MeiraGTx has successfully completed process performance qualification (PPQ). The Company has a commercial license from the UK Medicines and Healthcare products Regulatory Agency (MHRA) for its London manufacturing facility, as well as a commercial license for the Company's QC facility in Shannon, Ireland where release and stability assays for the product are conducted.
- MeiraGTx is now working to complete regulatory submissions in the U.S., EU, UK and Japan.

The U.S. Food and Drug Administration (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.

Clinical and Technology Programs

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

- In April 2026, MeiraGTx reported positive three-year data from its Phase 1 AQUAx clinical trial (n=24) evaluating AAV2-hAQP1 for the treatment of moderate to severe grade 2/3 radiation-induced xerostomia.
- Results demonstrated sustained, clinically meaningful improvements in both patient-reported outcomes and objective measures of salivary flow, with durable effects maintained from 12 months through 36 months post-treatment.
- AAV2-hAQP1 continued to be safe and well-tolerated at each dose tested.
- The results were presented on April 16, and a replay is available on the Investors page of the Company's website at investors.meiragtx.com.
- These findings provide strong clinical validation of the Company's salivary gland gene therapy platform and support continued advancement of this treatment not only in the pivotal AQUAx2 study, but also development in additional conditions where dry mouth is a significant patient burden.
- The Phase 2 AQUAx2 (NCT05926765) randomized, double-blind, placebo-controlled study at 30 sites in the U.S., Canada and the U.K. is closing this month with the 12-month pivotal data readout on track for the second quarter of 2027 which, if positive, would support a BLA filing and potential approval targeted for the end of 2027, with U.S. launch early in 2028.

FDA Breakthrough Therapy Designation (BTD) for AAV2-hAQP1:

- The U.S. Food and Drug Administration granted BTD to AAV2-hAQP1 for the treatment of grade 2 and grade 3 radiation-induced xerostomia caused by radiotherapy for cancers of the upper aerodigestive tract in March 2026.
- The designation was supported by 3-year data from the 24 patient Phase 1 AQUAx study in long term moderate to severe radiation induced xerostomia.
- BTD is for serious or life-threatening conditions and enables enhanced engagement with the FDA and potential for priority review.

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

- FDA granted Regenerative Medicine Advanced Therapy (RMAT) designation to AAV-GAD for the treatment of Parkinson's disease not adequately controlled with medication in 2025.
- This RMAT was awarded based on data demonstrating statistically significant efficacy in 2 double-blind sham-surgery controlled studies, a Phase 2 study (n=45), and a Phase 1/2 clinical bridging study (n=14) following the successful Phase 1 dose escalation study (n=14).
- This application also included the use of novel AI developed by our JV partner, Hologen, which demonstrated potential disease modification resulting from treatment.
- The Company is currently engaging with clinical trial sites globally and expects to initiate the Phase 3 study of AAV-GAD in the coming months.

AAV-AIPL1 for LCA4:

- MeiraGTx entered into a strategic collaboration with Lilly, granting Lilly worldwide exclusive rights to meduretgene parvec, or medu-vec (formerly referred to as AAV-AIPL1) program for Leber congenial amaurosis 4 (LCA4).
- Under the terms of the agreement, Lilly also received worldwide exclusive access rights to MeiraGTx's innovative gene therapy technologies for use in ophthalmology with certain targets designated by Lilly, including novel intravitreal capsids developed in-house at MeiraGTx and bespoke promoters including AI-generated cell specific promoters.
- MeiraGTx also granted Lilly certain rights to its proprietary riboswitch technology for use in gene editing in the eye.
- MeiraGTx received an upfront payment of $75 million and is eligible to receive over $400 million in total milestone payments. MeiraGTx is also eligible to receive tiered royalties on licensed products.

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

- The Company's Riboswitch technology is a powerful platform that transforms the potential of biologic therapeutics by providing a broadly applicable mechanism for the precise dosing of any protein, hormone or peptide that is encoded by DNA via *in vivo* production in direct dose response to bespoke oral small molecule inducers.
- MeiraGTx is progressing its first riboswitch program into the clinic in metabolic disease with native human leptin (Ribo-Leptin).
- The Company is in iterative discussion with the FDA to open a Ribo-Leptin IND later this year.
- The Company is also in IND enabling studies for a second riboswitch regulated vector for neuropathic pain.

Strengthened Balance Sheet with $100 Million Financing:

- In April 2026, MeiraGTx announced the pricing of an underwritten offering of 11,111,111 of its ordinary shares at an offering price of $9.00 per share, generating gross proceeds of approximately $100 million.

As of March 31, 2026, MeiraGTx had cash and cash equivalents of approximately $71.5 million. Based on the cash and cash equivalents and tax incentive receivable of $14.7 million, together with the approximately $100.0 million gross proceeds from the public equity offering in the second quarter of 2026 and the remaining $95.0 million upfront payment due from Hologen and associated reimbursements, the Company estimates that such funds will be sufficient to enable it to fund its operating expenses and capital expenditure requirements into the second half of 2028, including the $25.0 million upfront cash payment to J&J for the reacquisition of bota-vec and the repayment of its debt obligation to Perceptive Credit Holdings III, LP of $25.0 million (due in June 2026) and $50.0 million (due in July 2027).

For more information related to our clinical trials, please visit www.clinicaltrials.gov

Financial Results

Cash, cash equivalents and restricted cash were $73.8 million as of March 31, 2026, compared to $68.2 million as of December 31, 2025.

Service revenue was $0.3 million for the three months ended March 31, 2026, compared to $1.9 million for the three months ended March 31, 2025. The decrease of $1.6 million was due to decreased activity of PPQ services under the original asset purchase agreement with J&J as the work was substantially completed in the first half of 2025.

Cost of service revenue was $0.2 million for the three months ended March 31, 2026, compared to $1.4 million for the three months ended March 31, 2025. The decrease of $1.2 million was due to decreased activity of PPQ services under the original asset purchase agreement with J&J as the work was substantially completed in the first half of 2025.

General and administrative expenses were $8.9 million for the three months ended March 31, 2026, compared to $9.3 million for the three months ended March 31, 2025. The decrease of $0.4 million was primarily due to lower personnel related costs, including a decrease in payroll expense primarily due to lower bonus accruals, as well as a decrease in share-based compensation expense due to vesting in prior periods and a decrease in facilities costs. These decreases were partially offset by an increase in professional services costs. In addition, the three months ended March 31, 2025 included a release of asset retirement obligation provisions related to U.S. office and laboratory leases, which did not recur during the three months ended March 31, 2026.

Research and development expenses were $32.0 million for the three months ended March 31, 2026, compared to $32.8 million for the three months ended March 31, 2025. The decrease of $0.8 million was primarily due to decreases in other ocular diseases and AAV2-hAQP1 clinical programs, as there were no clinical trial material batches manufactured during the three months ended March 31, 2026 and overall lower clinical trial-related spend for AAV-GAD and AAV2-hAPQ1. In addition, costs associated with our preclinical programs for gene regulation and neurodegenerative diseases decreased compared to the prior year, primarily due to the completion of certain preclinical studies in 2025. These decreases were partially offset by an increase in manufacturing costs as there was no clinical trial material batch costs to allocate to our clinical programs as no batches were produced during the three months ended March 31, 2026, as well as a lower allocation of costs to cost of service revenue reflecting PPQ services provided under the original asset purchase agreement and related agreements being substantially completed during the first half of 2025.

Foreign currency loss was $2.8 million for the three months ended March 31, 2026, compared to a gain of $3.7 million for the three months ended March 31, 2025. The change of $6.5 million was primarily due to the strengthening 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 was $0.2 million for the three months ended March 31, 2026, compared to $1.0 million for the three months ended March 31, 2025. The decrease of $0.8 million was due to lower interest rates and cash balances held in interest bearing accounts during 2026.

Interest expense was $2.8 million for the three months ended March 31, 2026 compared to $3.0 million for the three months ended March 31, 2025. The decrease of $0.2 million was primarily due to a lower interest rate in connection with the debt financing.

Net loss attributable to ordinary shareholders for the quarter ended March 31, 2026, was $46.3 million, or $0.57 basic and diluted net loss per ordinary share, compared to a net loss attributable to ordinary shareholders of $40.0 million, or $0.51 basic and diluted net loss per ordinary share for the quarter ended March 31, 2025.

About MeiraGTx

MeiraGTx (Nasdaq: MGTX) is a vertically integrated, clinical-stage genetic medicines company with a broad pipeline with four late-stage clinical programs. Each of these programs use local delivery of small doses resulting in disease modifying effects in both inherited and more common diseases, in the eye, Parkinson's disease and radiation-induced xerostomia. MeiraGTx uses its innovative technology in optimization of capsids, promoters and novel translational control elements to develop best in class, potent, safe viral vectors. MeiraGTx's broad pipeline is supported by end-to-end in-house manufacturing. MeiraGTx has built the most comprehensive manufacturing capabilities in the industry, with 5 facilities globally, including two that are licensed for GMP viral vector production and a GMP QC facility with clinical and commercial licensure. In addition, MeiraGTx has developed a proprietary manufacturing platform process over 9 years based on more than 20 different viral vectors with leading yield and quality

aspects and commercial readiness. Uniquely, MeiraGTx has developed a novel technology for *in vivo* delivery of any biologic therapeutic using oral small molecules. This transformative riboswitch gene regulation technology allows precise, dose-responsive control of gene expression by oral small molecules. MeiraGTx is focusing the riboswitch platform on the regulated *in vivo* delivery of metabolic peptides, including GLP-1, GIP, Glucagon, Amylin, PYY and Leptin, as well as cell therapy, CAR-T for liquid and solid tumors and autoimmune diseases, and additionally PNS targets addressing long term intractable pain. MeiraGTx has developed the technology to apply genetic medicine to common diseases, increasing efficacy, addressing novel targets, and expanding access in some of the largest disease areas where the unmet need remains high.

For more information, please visit www.meiragtx.com

Forward Looking Statement

This press release contains forward-looking statements within the meaning of the Private Securities Litigation Reform Act of 1995. All statements contained in this press release that do not relate to matters of historical fact should be considered forward-looking statements, including, without limitation, statements regarding our product candidate development and anticipated milestones regarding our pre-clinical and clinical data, reporting of such data and the timing of results of data and regulatory matters, statements regarding our collaborations, as well as statements that include the words “expect,” “will,” “intend,” “plan,” “believe,” “project,” “forecast,” “estimate,” “may,” “could,” “should,” “would,” “continue,” “anticipate,” “eligible” and similar statements of a future or forward-looking nature. These forward-looking statements are based on management’s current expectations. These statements are neither promises nor guarantees, but involve known and unknown risks, uncertainties and other important factors that may cause actual results, performance or achievements to be materially different from any future results, performance or achievements expressed or implied by the forward-looking statements, including, but not limited to, our incurrence of significant losses; any inability to achieve or maintain profitability, raise additional capital, repay our debt obligations, identify additional and develop existing product candidates, successfully execute strategic transactions or priorities, bring product candidates to market, expansion of our manufacturing facilities and processes, successfully enroll patients in and complete clinical trials, accurately predict growth assumptions, recognize benefits of any orphan drug or rare pediatric disease designations, retain key personnel or attract qualified employees, or incur expected levels of operating expenses; the impact of pandemics, epidemics or outbreaks of infectious diseases on the status, enrollment, timing and results of our clinical trials and on our business, results of operations and financial condition; failure of early data to predict eventual outcomes; failure to obtain FDA or other regulatory approval for product candidates within expected time frames or at all; the novel nature and impact of negative public opinion of gene therapy; failure to comply with ongoing regulatory obligations; contamination or shortage of raw materials or other manufacturing issues; changes in healthcare laws; risks associated with our international operations; significant competition in the pharmaceutical and biotechnology industries; dependence on third parties; risks related to intellectual property; changes in tax policy or treatment; our ability to utilize our loss and tax credit carryforwards; litigation risks; and the other important factors discussed under the caption “Risk Factors” in our Quarterly Report on Form 10-Q for the quarter ended March 31, 2026, as such factors may be updated from time to time in our other filings with the SEC, which are accessible on the SEC’s website at www.sec.gov. These and other important factors could cause actual results to differ materially from those indicated by the forward-looking statements made in this press release. Any such forward-looking statements represent management’s estimates as of the date of this press release. 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 press release.

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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 March 31,	
	2026	2025
Revenues:		
Service revenue - related party	$ 293	$ 1,926
Total revenue	293	1,926
Operating expenses:		
Cost of service revenue - related party	198	1,378
General and administrative	8,928	9,364
Research and development	31,984	32,780
Total operating expenses	41,110	43,522
Loss from operations	(40,817)	(41,596)

Other non-operating income (expense):		
Foreign currency (loss) gain	(2,837)	3,687
Interest income	189	971
Interest expense	(2,848)	(3,043)
Net loss	(46,313)	(39,981)
Other comprehensive gain (loss):		
Foreign currency translation gain (loss)	172	(1,347)
Comprehensive loss	$ (46,141)	$ (41,328)
Net loss	$ (46,313)	$ (39,981)
Basic and diluted adjusted net loss per ordinary share	$ (0.57)	$ (0.51)
Weighted-average number of ordinary shares outstanding	81,300,944	79,032,341

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

	March 31, 2026	December 31, 2025
ASSETS		
CURRENT ASSETS:		
Cash and cash equivalents	$ 71,541	$ 65,931
Accounts receivable - related party	3,263	3,000
Prepaid expenses	6,031	6,017
Tax incentive receivable	14,696	15,286
Other current assets	670	1,527
Total Current Assets	96,201	91,761
Property, plant and equipment, net	102,573	105,465
Intangible assets, net	494	578
Restricted cash	2,217	2,262
Other assets	1,466	1,147
Equity method and other investments	6,749	6,749
Right-of-use assets - operating leases, net	12,124	12,852
Right-of-use assets - finance leases, net	22,831	23,616
TOTAL ASSETS	$ 244,655	$ 244,430
LIABILITIES AND SHAREHOLDERS' EQUITY		
CURRENT LIABILITIES:		
Accounts payable	$ 16,658	$ 10,066
Accrued expenses	25,239	32,893
Lease obligations - operating leases, current	2,221	2,851
Lease obligations - finance leases, current	39	38
Deferred revenue - related party, current	1,996	1,776
Note payable, net, current	24,866	24,648
Other current liabilities	105,108	50,283
Total Current Liabilities	176,127	122,555
Deferred revenue - related party	64,840	65,120
Lease obligations - operating leases	10,611	11,351
Lease obligations - finance leases	97	109
Asset retirement obligations	1,411	1,399
Note payable, net	49,699	49,689
TOTAL LIABILITIES	302,785	250,223
COMMITMENTS AND CONTINGENCIES		
SHAREHOLDERS' DEFICIT:		
Ordinary Shares, $0.00003881 par value, 1,288,327,750 authorized, 81,446,126 and 81,120,931 shares issued and outstanding at March 31, 2026 and December 31, 2025, respectively	3	3

Capital in excess of par value	820,018	808,021
Treasury shares	(18,193)	—
Accumulated other comprehensive gain	2,578	2,406
Accumulated deficit	(862,536)	(816,223)
Total Shareholders' Deficit	(58,130)	(5,793)
TOTAL LIABILITIES AND SHAREHOLDERS' DEFICIT	$ 244,655	$ 244,430