MeiraGTx Announces FDA Breakthrough Therapy Designation for AAV2-hAQP1 for the Treatment of Grade 2 and Grade 3 Radiation-Induced Xerostomia (RIX) and Reports Fourth Quarter and Full Year 2025 Financial and Operational Results

March 26, 2026

- *FDA granted Breakthrough Therapy Designation for AAV2-hAQP1 for the treatment of Grade 2 and Grade 3 late xerostomia caused by radiotherapy for cancers of the upper aerodigestive tract*

- *MeiraGTx to hold a program update and present long-term data for AAV2-hAQP1 program for the treatment of Grade 2/3 Radiation-Induced Xerostomia on Thursday, April 16th, 2026*

LONDON and NEW YORK, March 26, 2026 (GLOBE NEWSWIRE) -- MeiraGTx Holdings plc (Nasdaq: MGTX), a vertically integrated, clinical stage genetic medicines company, today announced financial and operational results for the fourth quarter and full-year ended December 31, 2025, and provided a corporate update.

“We are delighted to have been awarded Breakthrough Designation for our AAV2-hAQP1 treatment for Grade 2 and Grade 3 late xerostomia caused by radiotherapy for cancers of the upper aerodigestive tract,” said Alexandria Forbes, Ph.D., president and chief executive officer of MeiraGTx. “This Breakthrough application was supported by 3-year data from the Phase 1 dose escalation study. On April 16th, we will be providing an AAV2-hAQP1 program update with information about the commercial opportunity for this therapy, as well as presenting the 3-year data. We have also had huge enthusiasm about our Phase 2 AQUAx2 study in the RIX community, amongst physicians and patients.”

“In 2025 we executed two important strategic collaborations bringing immediate non-dilutive financing into the company, as well as potential significant near-term financial milestones. We signed a collaboration with Eli Lilly and Company (Lilly) focused on our AAV-AIPL1 program for the treatment of LCA4, one of the most severe forms of inherited retinopathies. In addition to AAV-AIPL1, Lilly gained exclusive rights to two preclinical ocular programs as well as our intravitreal capsids, bespoke promoters and certain rights to our riboswitch platform in the eye. Lilly is working with global regulatory agencies to expeditiously gain approval of AAV-AIPL1 and to provide access to this life changing therapy for LCA4 to children globally.”

Dr. Forbes continued, “Earlier in the year, we entered into a strategic collaboration with Hologen Limited, a world-leader in the development of multi-modal generative AI foundation models which were built specifically to remove noise from clinical data and to allow the real clinical effects of treatment to be clearly seen. The use of Hologen’s AI technology applied to MeiraGTx’s statistically significant double-blind Phase 2 data-sets has de-risked the AAV-GAD program and identified disease modifying changes in the physiology of the brain in response to treatment. We are working closely with Hologen to initiate the pivotal Phase 3 double blind sham-controlled study of AAV-GAD in Parkinson’s in the coming months at global centers of excellence in Parkinson’s disease treatment.”

“We have also been successful in the further development of our Riboswitch platform. We are currently in conversation with the FDA preparing our first Riboswitch IND with our Ribo-leptin product to deliver leptin using a daily oral small molecule inducer. We have very strong long-term data in animal models demonstrating the small molecule controlled riboswitch dynamics are durable for the life of the animal – out to 19 months so far. We have also now demonstrated very encouraging data in the second Riboswitch program that we intend to take into the clinic which is in neuropathic pain.”

2025 and Recent Highlights

AAV2-hAQP1 for the Treatment of Radiation-Induced Xerostomia (RIX):

- The FDA has now granted Breakthrough Therapy Designation for AAV2-hAQP1 for the treatment of Grade 2 and Grade 3 late xerostomia caused by radiotherapy for cancers of the upper aerodigestive tract.
- This is in addition to the Regenerative Medicine Advanced Therapy (RMAT) designation already granted by the FDA for AAV2-hAQP1.
- The Company has aligned with the FDA on the clinical requirements for the Phase 2 AQUAx2 (NCT05926765) study to support a potential BLA with the primary endpoint being the change from baseline in the Xerostomia Questionnaire at 12 months following the one time treatment.
- The final patients are currently enrolling, and the Company anticipates data 12 months after the last patient is treated, with a potential BLA filing in the first half of 2027 and potential approval around the end of 2027 with launch in the US targeted in early 2028.
- MeiraGTx will be hosting a program update April 16th to discuss the commercial opportunity as well as presenting the full 3-year data from all cohorts of the Phase 1 study.

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

- In 2025, the FDA granted RMAT designation to AAV-GAD for the treatment of Parkinson’s disease not adequately

controlled with medication.
- This RMAT was awarded based on positive data demonstrating statistically significant efficacy in 3 clinical studies: a Phase 1 dose escalation study (n=14), a double-blind sham-surgery controlled Phase 2 study (n=45), and a double-blind sham-surgery controlled Phase 1/2 clinical bridging study (n=14). This application also included the demonstration of potential disease modification resulting from treatment in the Company's positive Phase 2 studies.
- 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**.**

Strategic Collaboration with Hologen AI:

- MeiraGTx and Hologen have formed a joint venture, Hologen Neuro AI Ltd, with a $200 million upfront payment to MeiraGTx, as well as additional committed funding from Hologen into the joint venture of up to $230 million to fully fund the development of the AAV-GAD program through approval.
- MeiraGTx will hold a 30% ownership in the joint venture and lead all clinical development and manufacturing.
- Hologen Neuro AI Ltd will contribute its proprietary multi-modal generative foundation models (LMMs) to the joint venture and will enter into both clinical and commercial manufacturing supply agreements with MeiraGTx for exclusive manufacturing of AAV-GAD.
- As part of the Hologen collaboration, the Company also intends to move forward into the clinic this year with a locally delivered treatment for trigeminal neuralgia, one of the most severe forms of pain and intractable to treatment.

Ophthalmology Programs
Strategic partnership with Eli Lilly and Company on AAV-AIPL1 for LCA4

- MeiraGTx entered into a strategic collaboration with Lilly, granting Lilly worldwide exclusive rights to the AAV-AIPL1 program for Leber congenial amaurosis 4 (LCA4) and access to additional ocular and gene regulation assets. Under the terms of the agreement, 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.
- 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.

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.
- 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 with MeiraGTx eligible to receive up to $285 million upon the first commercial sales of bota-vec in the US and EU and manufacturing tech transfer.
- MeiraGTx also entered into a commercial supply agreement with Johnson & Johnson Innovative Medicine for bota-vec manufacturing. As part of this agreement, MeiraGTx has completed PPQ to support CMC sections of global regulatory filings.
- 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.

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.
- AI driven target discovery is identifying a universe of peptides, hormones and proteins with important roles in homeostatic pathways regulating cardiovascular, metabolic, neurological and immunological systems that underly many of the diseases of aging.
- Such proteins acting in rapidly responsive systems are often short lived and hard to make into long-acting injectable analogs that retain full physiological function.
- The Company's Riboswitch technology provides the only broadly applicable mechanism for precisely dosing the growing number of proteins that are currently intractable to use as therapeutics.
- MeiraGTx is progressing its first riboswitch program into the clinic in metabolic disease with native human leptin (Ribo-

leptin).

- This is a significant unmet need in patients with both inherited and acquired leptin deficiency. The only currently available treatment - metreleptin - is immunogenic, which can lead to neutralizing antibodies against leptin, resulting catastrophic and even lethal metabolic consequences.
- The Company is in iterative discussion with the FDA to open a Ribo-leptin IND later this year.

As of December 31, 2025, MeiraGTx had cash and cash equivalents of approximately $65.9 million, as well as $3.0 million in receivables due from Johnson & Johnson Innovative Medicine and $15.3 million in tax incentive receivables. Together with the $55.0 million received to date in the first quarter 2026 and $5.0 million in receivables from Hologen as well as the remaining $95.0 million from the closing of the strategic collaboration with Hologen, the Company believes that it will have sufficient capital to fund operating expenses and capital expenditure requirements into the second half of 2027 and to repay its debt obligation of $25.0 million to Perceptive Credit Holdings III, LP (due in June 2026) and $50.0 million (due in May 2027). This estimate does not include the $135.0 million in potential near-term cash consideration from Lilly upon the achievement of certain development and regulatory approval milestones, or the $285.0 million in milestones the Company is eligible to receive under the asset purchase agreement upon first commercial sale of bota-vec 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 bota-vec.

Financial Results

Cash, cash equivalents and restricted cash were $68.2 million as of December 31, 2025, compared to $105.7 million as of December 31, 2024.

Service revenue was $6.4 million for the year ended December 31, 2025, compared to $33.3 million for the year ended December 31, 2024. The decrease of $26.9 million was due to decreased activity of PPQ services under the asset purchase agreement with Johnson & Johnson Innovative Medicine as the work was substantially completed during the first half of 2025.

License revenue was $75.0 million for the year ended December 31, 2025 due to the upfront license fee payment under the Lilly collaboration agreement. There was no license revenue for the year ended December 31, 2024.

Cost of service revenue was $4.8 million for the year ended December 31, 2025, compared to $23.8 million for the year ended December 31, 2024. The decrease of $18.9 million was due to decreased activity of PPQ services under the asset purchase agreement with Johnson & Johnson Innovative Medicine as the work was substantially completed during the first half of 2025.

General and administrative expenses were $52.9 million for the year ended December 31, 2025, compared to $54.2 million for the year ended December 31, 2024. The decrease of $1.3 million was primarily due to a decrease in professional fees, legal fees, a change in estimate of an asset retirement obligation, which were partially offset by an increase in payroll expenses and facilities costs.

Research and development expenses for the year ended December 31, 2025 were $129.6 million, compared to $119.5 million for the year ended December 31, 2024. The increase of $10.1 million was primarily due to an increase in manufacturing costs due to both a lower allocation of clinical trial material batch costs to our clinical programs and a lower allocation of costs to cost of services revenue reflecting PPQ services provided under the Asset Purchase Agreement and related agreements being substantially completed during the first half of 2025. Other cost increases arose in our clinical programs for other ocular diseases and AAV-GAD, primarily due to an increase in manufactured clinical trial material batches related to these programs, and our preclinical programs for gene regulation reflecting preclinical studies initiated during the year. These increases were partially offset by a decrease in costs for our AAV-hAQP1 program due to a decrease in the number of batches of clinical trial material manufactured compared to the prior year.

Foreign currency gain was $2.1 million for the year ended December 31, 2025, compared to a loss of $2.9 million for the year ended December 31, 2024. The change of $5.0 million was primarily due to the weakening of the U.S. dollar against the pound sterling and euro as it mostly relates to the valuation of our intercompany payables and receivables.

Interest income was $1.8 million for the year ended December 31, 2025, compared to $4.1 million for the year ended December 31, 2024. The decrease of $2.3 million was due to lower interest rates and cash balances during 2025.

Interest expense was $12.2 million for the year ended December 31, 2025, compared to $13.3 million for the year ended December 31, 2024. The decrease of $1.1 million was primarily due to a lower interest rate in connection with the debt financing.

There was no gain on sale of nonfinancial assets during the year ended December 31, 2025 compared to $28.4 million for the year ended December 31, 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 being fully recognized during 2023 and 2024.

Net loss attributable to ordinary shareholders for the year ended December 31, 2025, was $114.2 million, or $1.42 basic and diluted net loss per ordinary share, compared to a net loss attributable to ordinary shareholders of $147.8 million, or $2.12 basic and diluted net loss per ordinary share for the year ended December 31, 2024.

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

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, potential milestone payments and the achievement of such milestones, statements regarding our collaborations, including the anticipated timing for the closing and funding of the collaboration with Hologen, the success of the activities to be performed under the Hologen collaboration agreements and the efficacy of Hologen's AI technology, the development of our AAV-GAD and other CNS product candidates and the development of our manufacturing technology, 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 Annual Report on Form 10-K for the year ended December 31, 2025, 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
CONSOLIDATED STATEMENTS OF OPERATIONS AND COMPREHENSIVE LOSS
(in thousands, except share and per share amounts)

	For the Years Ended December 31,	
	2025	2024
Revenues:		
Service revenue - related party	$ 6,400	$ 33,279
License revenue	74,991	—
Total revenue	81,391	33,279
Operating expenses:		
Cost of service revenue - related party	4,843	23,791
General and administrative	52,897	54,216
Research and development	129,619	119,484
Total operating expenses	187,359	197,491

Loss from operations	(105,968)	(164,212)
Other non-operating income (expense):		
Foreign currency gain (loss)	2,146	(2,886)
Interest income	1,818	4,145
Interest expense	(12,197)	(13,272)
Gain on sale of nonfinancial assets	—	28,434
Net loss	(114,201)	(147,791)
Other comprehensive gain (loss):		
Foreign currency translation gain (loss)	6,125	(2,284)
Comprehensive loss	$ (108,076)	$ (150,075)
Net loss	$ (114,201)	$ (147,791)
Basic and diluted net loss per ordinary share	$ (1.42)	$ (2.12)
Weighted-average number of ordinary shares outstanding	80,430,186	69,822,353

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

	December 31, 2025	December 31, 2024
ASSETS		
CURRENT ASSETS:		
Cash and cash equivalents	$ 65,931	$ 103,659
Accounts receivable - related party	3,000	707
Contract assets - related party	—	950
Inventory	—	385
Prepaid expenses	6,017	6,828
Tax incentive receivable	15,286	8,971
Other current assets	1,527	2,018
Total Current Assets	91,761	123,518
Property, plant and equipment, net	105,465	102,878
Intangible assets, net	578	821
Restricted cash	2,262	2,009
Other assets	1,147	1,002
Equity method and other investments	6,749	6,749
Right-of-use assets - operating leases, net	12,852	10,576
Right-of-use assets - finance leases, net	23,616	22,198
TOTAL ASSETS	$ 244,430	$ 269,751
LIABILITIES AND SHAREHOLDERS' (DEFICIT) EQUITY		
CURRENT LIABILITIES:		
Accounts payable	$ 10,066	$ 23,586
Accrued expenses	32,893	27,414
Lease obligations - operating leases, current	2,851	4,053
Lease obligations - finance leases, current	38	—
Deferred revenue - related party, current	1,776	4,827
Note payable, net, current	24,648	—
Other current liabilities	50,283	903
Total Current Liabilities	122,555	60,783
Deferred revenue - related party	65,120	57,576
Lease obligations - operating leases	11,351	7,523
Lease obligations - finance leases	109	—
Asset retirement obligations	1,399	2,821
Note payable, net	49,689	73,221
TOTAL LIABILITIES	250,223	201,924
COMMITMENTS AND CONTINGENCIES (Note 15)		
SHAREHOLDERS' (DEFICIT) EQUITY:		

Ordinary Shares, $0.00003881 par value, 1,288,327,750 authorized, 81,120,931 and 78,397,380 shares issued and outstanding at December 31, 2025 and 2024, respectively	3	3
Capital in excess of par value	808,021	773,565
Accumulated other comprehensive gain (loss)	2,406	(3,719)
Accumulated deficit	(816,223)	(702,022)
Total Shareholders' (Deficit) Equity	(5,793)	67,827
TOTAL LIABILITIES AND SHAREHOLDERS' (DEFICIT) EQUITY	$ 244,430	$ 269,751