MeiraGTx Reports Third Quarter 2025 Financial and Operational Results

November 13, 2025

- *Entered into broad strategic collaboration with Eli Lilly and Company ("Lilly") in the area of ophthalmology, granting Lilly worldwide exclusive rights to the Company's AAV-AIPL1 program for treatment of one of the most severe inherited retinopathies, Leber congenital amaurosis 4 (LCA4)*

- *Released material under the Company's Specials license for a second rare pediatric ophthalmology condition caused by mutations in BBS10, with the prescribing physician treating the first patient during the quarter. The BBS10 program has been awarded Rare Pediatric Disease Designation (RPDD)*

LONDON and NEW YORK, Nov. 13, 2025 (GLOBE NEWSWIRE) -- MeiraGTx Holdings plc (Nasdaq: MGTX), a vertically integrated, clinical-stage genetic medicines company, today announced financial and operational results for the third quarter ended September 30, 2025, and provided a corporate update.

"Following the close of the third quarter, we are very pleased to have signed a strategic collaboration in ophthalmology with Eli Lilly, led by our AAV-AIPL1 program for the treatment of LCA4, one of the most severe forms of inherited retinopathies," said Alexandria Forbes, Ph.D., president and chief executive officer of MeiraGTx. "The unprecedented data from the 11 LCA4 children born blind who all gained vision after treatment with AAV-AIPL1 illustrates the power of gene therapy in the eye to cure even the most severe genetic defects, particularly when treatment takes place at a very young age. In addition to AAV-AIPL1, Lilly has gained rights to two preclinical ocular programs as well as our intravitreal capsids, bespoke promoters generated from our AI driven promoter platform, and certain rights to our riboswitch platform in the eye. We are excited to be entering into this collaboration with Lilly and view this as a testament to the power of our broad toolkit of proprietary gene therapy technologies for both rare and prevalent ocular disease. We are particularly pleased that Lilly, a global leader in development and commercialization of innovative medicines, has chosen to partner with us in this area of high unmet need, and shares our dedication to bringing truly life changing therapies to patients with otherwise intractable conditions."

Dr. Forbes continued, "During the quarter we continued to make progress in our late-stage clinical programs. Our pivotal Phase 2 study of AAV-hAQP1 for the treatment of grade 2 or 3 radiation-induced xerostomia (RIX) remains on track to achieve target enrollment at the end of this year, and we expect to have data which if positive may lead to a BLA for AAV-hAQP1 in early 2027, with potential approval later in 2027. We also anticipate initiating a Phase 3 study evaluating AAV-GAD for the treatment of Parkinson's disease in the coming months and are currently in discussion with sites globally for inclusion in the Phase 3 study. This will be our third double-blind, sham-controlled clinical study of AAV-GAD where we aim to demonstrate significant improvement in UPDRS as well as the potential disease-modifying effect of AAV-GAD treatment on the physiological circuitry controlling movement as well as a potentially protective effect on the substantia nigra."

"In addition," said Dr. Forbes, "we have completed optimization of our lead riboswitch program for entry into the clinic, for the delivery of native human leptin controlled by a daily oral small molecule to treat inherited and acquired leptin deficiency. This is an important unmet need due to the immunogenicity of the only available current treatment, metreleptin, a synthetic injectable leptin analog which can result in neutralizing antibody production with catastrophic or even lethal metabolic consequences. We have now demonstrated complete durability of both leptin production dynamics and efficacy of our riboswitch-controlled leptin over more than a year of oral dosing in the mouse model. We have manufactured GMP small molecule inducer for the clinic and we are engaging the FDA in IND enabling discussions."

"And finally, we have successfully developed, manufactured and provided material for our second Specials program in the UK for the treatment of BBS10-associated retinal dystrophy. Like AAV-AIPL1, this program is in collaboration with a BBS10 philanthropic organization. One child has been treated so far and others may be treated if they are found to be eligible by the treating physician. The BBS10 program has been awarded Rare Pediatric Disease Designation by the FDA, making it potentially eligible for a Priority Review Voucher on approval," Dr. Forbes stated.

Recent Development Highlights

Strategic Collaboration with Eli Lilly and Company to Develop and Commercialize Genetic Medicines in Ophthalmology:

- In November 2025, the Company announced a broad strategic collaboration in the area of ophthalmology with Eli Lilly and Company ("Lilly"), granting Lilly worldwide exclusive rights to its AAV-AIPL1 program for treatment of one of the most severe inherited retinopathies, Leber congenital amaurosis 4 (LCA4) owing to genetic deficiency of Aryl-hydrocarbon-interacting protein-like 1 (AIPL1).
- Lilly also receives worldwide exclusive access rights to MeiraGTx's innovative gene therapy technologies for use in ophthalmology with certain targets named by Lilly, including novel intravitreal capsids developed in-house at MeiraGTx and bespoke promoters including AI-generated promoters for specific cells in the retina.
- MeiraGTx also grants Lilly certain rights to its proprietary riboswitch technology for use in gene editing in the eye. MeiraGTx's riboswitch technology platform is broadly applicable to any therapeutic protein. It allows precise, titratable *in*

vivo production of the therapeutic protein or gene editing nuclease from a gene template controlled by oral dosing of a small molecule inducer.
- Under the terms of the agreement, MeiraGTx will receive an upfront payment of $75 million and will be eligible to receive over $400 million in total milestone payments. MeiraGTx is also eligible to receive tiered royalties on licensed products.

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

- In December 2024, MeiraGTx was granted Regenerative Medicine Advanced Therapy (RMAT) designation by the FDA for AAV2-hAQP1 for the treatment of Grade 2/3 RIX.
- Following FDA interactions through the RMAT meeting process, the Company has 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 at the end of the year, and the potential for pivotal data read out which if positive could potentially support a BLA filing in early 2027 with potential approval later in the year.
- 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 :

- 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.
- 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).
- The Company is currently engaging with sites globally with the aim of initiating the Phase 3 study of AAV-GAD in the coming months.

Strategic Collaboration with Hologen AI:

- The Company has received $50 million of the $200 million in upfront cash consideration due after receipt of clearance and approval under the foreign direct investment laws, with the remainder expected in the fourth quarter of 2025.
- With an additional 250,000 Class A shares of Hologen being granted to MeiraGTx in addition to the $200 million upfront cash consideration, the value of MeiraGTx's equity ownership in Hologen AI will be approximately $30 million.
- MeiraGTx and Hologen are forming a joint venture, Hologen Neuro AI Ltd, with the $200 million upfront payment and an additional committed funding from Hologen of up to $230 million into the joint venture to finance the development of the AAV-GAD program for the treatment of Parkinson's disease.
- The joint venture will use Hologen's proprietary multi-modal generative foundation models (LMMs).
- MeiraGTx 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 MeiraGTx for exclusive manufacturing of AAV-GAD and other locally-delivered genetic medicines targeting the CNS.
- Hologen will own a minority stake in MeiraGTx's 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 MeiraGTx's proprietary manufacturing capabilities.
- As part of the Hologen collaboration, the Company will move 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 the first half of 2026 using material manufactured in house at MeiraGTx.

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.
- MeiraGTx 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.
- MeiraGTx also entered into a commercial supply agreement with Johnson & Johnson Innovative Medicine for bota-vec manufacturing, which the Company anticipates will generate additional revenue during the product launch. As part of this commercial supply agreement, the Company has now completed PPQ to potentially support CMC sections of global regulatory filings.

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

- MeiraGTx is progressing its first riboswitch program into the clinic in metabolic disease with native human leptin produced in response to an oral small molecule. 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 with resulting catastrophic and even lethal metabolic consequences.
- The Ribo-Leptin construct has been optimized for the clinic for one-time intramuscular delivery. The small molecule inducer has been manufactured under GMP for the clinic, and the Company is in IND-enabling discussions with regulatory agencies.
- MeiraGTx has demonstrated very clear leptin production and efficacy in tight dose response to the oral small molecule inducer, and have now shown the durability of leptin production dynamics as well as efficacy in the mouse model unchanged out past 1 year of small molecule dosing.

As of September 30, 2025, MeiraGTx had cash and cash equivalents of approximately $14.8 million, as well as $2.8 million in receivables due from Johnson & Johnson Innovative Medicine and $9.0 million in tax incentive receivables. Together with the $75.0 million upfront cash payment from Lilly, and the $22.0 million received to date in the fourth quarter 2025 and the remaining $150.0 million from the anticipated 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 $75.0 million to Perceptive Credit Holdings III, LP (due in August 2026). 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 $17.1 million as of September 30, 2025, compared to $105.7 million as of December 31, 2024.

Service revenue was $0.4 million for the three months ended September 30, 2025, compared to $10.9 million for the three months ended September 30, 2024. The decrease of $10.5 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 as of September 30, 2025.

Cost of service revenue was $0.3 million for the three months ended September 30, 2025, compared to $12.0 million for the three months ended September 30, 2024. The decrease of $11.7 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 as of September 30, 2025.

General and administrative expenses were $13.6 million for the three months ended September 30, 2025, compared to $12.7 million for the three months ended September 30, 2024. The increase of $0.9 million was primarily due to an increase in rent and facilities costs, a loss on disposal of equipment, furniture and fixtures, and increases in consulting fees and share-based compensation. These increases were partially offset by a decrease in legal and accounting fees.

Research and development expenses for the three months ended September 30, 2025 were $32.5 million, compared to $26.2 million for the three months ended September 30, 2024. The increase of $6.3 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 service revenue reflecting PPQ services provided under the asset purchase agreement with Johnson and Johnson Innovative Medicine being substantially complete by September 30, 2025. Other cost increases arose in our clinical programs for other ocular diseases, 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 three months ended September 30, 2025. These increases were partially offset by a decrease in our AAV-hAQP1 program due to the required clinical trial material being substantially manufactured in the prior year.

Foreign currency loss was $1.6 million for the three months ended September 30, 2025, compared to a gain of $3.5 million for the three months ended September 30, 2024. The change of $5.1 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 was $0.2 million for the three months ended September 30, 2025, compared to $1.2 million for the three months ended September 30, 2024. The decrease of $1.0 million was due to lower interest rates and cash balances held in interest bearing accounts during 2025.

Interest expense was $3.1 million for the three months ended September 30, 2025, compared to $3.4 million for the three months ended September 30, 2024. The decrease of $0.3 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 September 30, 2025, was $50.5 million, or $0.62 basic and diluted net loss per ordinary share, compared to a net loss attributable to ordinary shareholders of $39.3 million, or $0.55 basic and diluted net loss per ordinary share for the quarter ended September 30, 2024.

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, the development of our manufacturing technology, 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, 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 September 30, 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
CONDENSED CONSOLIDATED STATEMENTS OF OPERATIONS AND COMPREHENSIVE LOSS
(unaudited)
(in thousands, except share and per share amounts)

	For the Three-Month Periods Ended September 30,		For the Nine-Month Periods Ended September 30,	
	2025	2024	2025	2024
Revenues:				
Service revenue - related party	$ 410	$ 10,910	$ 6,027	$ 11,889
Total revenue	410	10,910	6,027	11,889
Operating expenses:				
Cost of service revenue - related party	313	11,985	4,367	11,985
General and administrative	13,616	12,723	35,293	37,127
Research and development	32,532	26,243	98,807	95,499
Total operating expenses	46,461	50,951	138,467	144,611
Loss from operations	(46,051)	(40,041)	(132,440)	(132,722)
Other non-operating income (expense):				
Foreign currency (loss) gain	(1,558)	3,463	10,753	2,644
Interest income	161	1,189	1,540	3,113
Interest expense	(3,065)	(3,357)	(9,142)	(9,861)
(Loss) gain on sale of nonfinancial assets	—	(584)	—	28,434
Net loss	(50,513)	(39,330)	(129,289)	(108,392)
Other comprehensive loss:				
Foreign currency translation gain (loss)	1,213	(1,234)	(2,593)	(3,413)
Comprehensive loss	$ (49,300)	$ (40,564)	$ (131,882)	$ (111,805)
Net loss	$ (50,513)	$ (39,330)	$ (129,289)	$ (108,392)
Basic and diluted net loss per ordinary share	$ (0.62)	$ (0.55)	$ (1.61)	$ (1.62)
Weighted-average number of ordinary shares outstanding	80,947,703	71,633,150	80,195,572	66,709,847

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

	September30, 2025	December31, 2024
ASSETS		
CURRENT ASSETS:		
Cash and cash equivalents	$ 14,841	$ 103,659
Accounts receivable - related party	2,808	707
Contract assets - related party	—	950
Inventory	—	385
Prepaid expenses	7,195	6,828
Tax incentive receivable	9,002	8,971
Other current assets	328	2,018
Total Current Assets	34,174	123,518
Property, plant and equipment, net	107,653	102,878
Intangible assets, net	653	821
Restricted cash	2,265	2,009
Other assets	1,111	1,002
Equity method and other investments	6,749	6,749
Right-of-use assets - operating leases, net	13,064	10,576
Right-of-use assets - finance leases, net	23,802	22,198
TOTAL ASSETS	$ 189,471	$ 269,751

LIABILITIES AND SHAREHOLDERS' EQUITY		
CURRENT LIABILITIES:		
Accounts payable	$ 21,537	$ 23,586
Accrued expenses	23,872	27,414
Lease obligations, current	2,475	4,053
Deferred revenue - related party, current	2,742	4,827
Note payable, net, current	74,055	—
Other current liabilities	28,241	903
Total Current Liabilities	152,922	60,783
Deferred revenue - related party	64,210	57,576
Lease obligations	11,531	7,523
Asset retirement obligations	1,365	2,821
Note payable, net	—	73,221
TOTAL LIABILITIES	230,028	201,924
COMMITMENTS AND CONTINGENCIES (Note 11)		
SHAREHOLDERS' EQUITY (DEFICIT):		
Ordinary Shares, $0.00003881 par value, 1,288,327,750 authorized, 80,479,684 and 78,397,380 shares issued and outstanding at September 30, 2025 and December 31, 2024, respectively	3	3
Capital in excess of par value	797,063	773,565
Accumulated other comprehensive loss	(6,312)	(3,719)
Accumulated deficit	(831,311)	(702,022)
Total Shareholders' Equity	(40,557)	67,827
TOTAL LIABILITIES AND SHAREHOLDERS' EQUITY	$ 189,471	$ 269,751