MeiraGTx Reports Third Quarter 2026 Financial and Operational Results

November 12, 2026

- *Submission of Marketing Authorisation Application (MAA) to the European Medicines Agency (EMA) for botaretigene sparoparvovec (bota-vec) for the treatment of X-linked retinitis pigmentosa (XLRP) expected in December 2026*
- *Submission of full Biologics License Application (BLA) to the U.S. Food and Drug Administration (FDA) for bota-vec expected in the first quarter of 2027*
- *12-month data readout in pivotal Phase 2 AQUAx2 study of AAV2-hAQP1 for the treatment of radiation-induced xerostomia (RIX) mid-2027*
- *Upcoming oral presentation at ObesityWeek highlights AAV-BDNF program for the treatment of MC4R/BDNF genetic obesity (severe early-onset syndromic obesity)*
- *Strengthened leadership with the appointment of John Knighton as Executive Vice President of Global Manufacturing and Supply Chain and Alan M. Sebulsky to the Board of Directors*

LONDON and NEW YORK, Nov. 12, 2026 (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, 2026, and provided a corporate update.

“We are pleased to report that we have now completed much of the work towards submission of full global regulatory filings with the EMA and FDA for bota-vec for the treatment of XLRP. The MAA dossier is complete and awaiting submission and the BLA is expected to follow early next year,” said Alexandria Forbes, Ph.D., president and chief executive officer of MeiraGTx. “In parallel with these filings, we have been preparing our market access and commercial teams ahead of the anticipated launch of our first commercial product, bota-vec, in late 2027.”

Dr. Forbes continued, “We are actively preparing for our pivotal Phase 2 AQUAx2 12-month data readout for the treatment of radiation-induced xerostomia in mid-2027. As we progress these two lead programs through filing for potential approval and commercialization, we are also advancing several programs towards IND. Two of these programs use our transformative riboswitch platform: Ribo-Leptin for metabolic disease, and a novel target with riboswitch to treat neuropathic pain.”

“Other programs to highlight in the coming quarters, which we have spent years optimizing, are our dry AMD and Geographic Atrophy programs where we are advancing two novel targets,” said Dr. Forbes. “Using our internal and partnered AI technology, we have developed proprietary intravitreal capsids and strong cell specific promoters which can deliver a highly potent combination addressing both upstream and downstream pathways involved in the pathogenesis of dry AMD.”

Third Quarter 2026 and Recent Highlights

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

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

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

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

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

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

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

AAV-AIPL1 for LCA4:

- MeiraGTx has granted Eli Lilly and Company (Lilly) worldwide exclusive rights to AAV-AIPL1 for the treatment of Leber congenital amaurosis 4 (LCA4). Lilly also received exclusive access rights to certain of MeiraGTx's gene therapy technologies for ophthalmology, and certain rights to the Company's riboswitch technology for 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, as well as tiered royalties on licensed products.

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

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

Corporate and Leadership Updates

- In July 2026, MeiraGTx appointed John Knighton as Executive Vice President of Global Manufacturing and Supply Chain. Mr. Knighton brings more than 30 years of experience from J&J and GlaxoSmithKline. Most recently, he served as Vice

President, Cell & Gene Therapy API Development at Janssen Pharmaceuticals, Inc., where he led a team that manufactured for the approval and commercialization of CARVYKTI® (a personalized CAR T-cell immunotherapy used to treat adult patients with relapsed or refractory multiple myeloma).

- In September 2026, MeiraGTx appointed Alan M. Sebulsky to its Board of Directors. Mr. Sebulsky has more than 40 years of experience in public company investment, financial strategy, and board leadership, most recently serving as Partner and Portfolio Manager at Adage Capital Management for 14 years. He is the Managing Member of Apothecary Capital, serves on the Board of Directors of Parabilis Medicines, Inc., and previously served on the boards and audit committees of Jazz Pharmaceuticals and Arrow International.

Upcoming Oral Presentation at ObesityWeek Highlighting AAV-BDNF Program for the Treatment of MC4R/BDNF Genetic Obesity (severe early-onset syndromic obesity)
Session Details: Brain Biology, Gene Therapy, and Treatment Outcomes Oral Session
Monday November 16, 2026
8:30 AM- 9:30 AM ET
Title: *Restoration of the Leptin-Melanocortin Pathway via Hypothalamic AAV-BDNF Rescues Syndromic Obesity*
Speaker: Josefa Sullivan
Location: Gaylord National Resort & Convention Center: Potomac AB, Ballroom Level, National Harbor, Maryland

As of September 30, 2026, MeiraGTx had cash and cash equivalents of approximately $184.7 million as well as $1.7 million in accounts receivable, $3.8 million in accounts receivable – related party, $0.6 million in unbilled receivables, $17.2 million in unbilled receivables – related party and $6.2 million in tax incentive receivables. Together with the additional $95.0 million upfront payment due from Hologen and associated reimbursements, the Company believes that it will have sufficient capital to fund operating expenses and capital expenditure requirements into the second half of 2028. This estimate does not include the $135.0 million in potential near-term cash consideration from Lilly upon achievement of certain development and regulatory approval milestones, or any subsequent tranches available under the royalty note purchase agreement with Oberland Capital Management LLC (Oberland Capital).

Financial Results

Cash, cash equivalents and restricted cash were $186.8 million as of September 30, 2026, compared to $17.1 million as of September 30, 2025.

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

Service revenue – related party was $3.2 million for the three months ended September 30, 2026, compared to $0.4 million for the three months ended September 30, 2025. The increase of $2.8 million was primarily attributable to the development and transition services provided to Reogen under the Reogen collaboration agreement and related agreements in the current period, compared to PPQ services provided to J&J under the original asset purchase agreement and related agreements in 2025.

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

Cost of service revenue – related party was $2.8 million for the three months ended September 30, 2026 compared to $0.3 million for the three months ended September 30, 2025. The increase of $2.5 million was primarily attributable to costs incurred during the current period related to the development and transition services provided to Reogen under the Reogen collaboration agreement and related agreements, compared to costs associated with PPQ services provided to J&J under the original asset purchase agreement and related agreements in 2025.

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

Research and development expenses were $39.3 million for the three months ended September 30, 2026, compared to $32.5 million for the three months ended September 30, 2025. The increase of $6.8 million was primarily attributable to increased clinical trial activities and manufacturing of clinical trial batch material related to the AAV2-hAQP1 program, higher expenses associated with the bota-vec program following the reacquisition from Janssen in the second quarter of 2026, and increased costs associated with the AAV-GAD program, primarily due to the allocation of manufacturing batch costs that were not recognized in cost of service revenue – related party. Manufacturing costs also increased primarily due to scheduled maintenance activities during the quarter, although this increase was fully offset by lower expenses associated with other preclinical ocular programs. In addition, expenses associated with other clinical ocular programs decreased.

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

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

Financial related costs, net reflected a gain of $1.6 million for the three months ended September 30, 2026 compared to an expense of $3.1 million for

the three months ended September 30, 2025. The favorable change of $4.7 million was primarily due to a gain from the change in fair value of the royalty notes and lower interest expense following the termination of the notes purchase agreement with Perceptive Credit Holdings III, LP in the second quarter of 2026, partially offset by the transaction cost expensed in connection with the issuance of the royalty notes and subsequent change in fair value.

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

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

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

Net loss attributable to ordinary shareholders for the quarter ended September 30, 2026 was $51.3 million, or $0.53 basic and diluted net loss per ordinary share, compared to a net loss attributable to ordinary shareholders of $50.5 million, or $0.62 basic and diluted net loss per ordinary share for the quarter ended September 30, 2025.

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, radiation-induced xerostomia, and Parkinson's disease. 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, including two facilities 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 10 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 timing of regulatory submissions and the potential approval and commercial launch of our product candidates, our ability to manufacture product candidates, and 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 September 30, 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) INCOME
(unaudited)
(in thousands, except share and per share amounts)

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

CONDENSED CONSOLIDATED BALANCE SHEETS
(unaudited)
(in thousands, except share and per share amounts)

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