MeiraGTx Announces Third Quarter 2024 Financial and Operational Results and Recent Business Updates

November 13, 2024

- *Received 3 Rare Pediatric Disease Designations (RPDD) from FDA for each of 3 potential therapies for 3 different rare inherited retinopathies including AAV-AIPL1*
- *Agreed on pathway with MHRA for Marketing Authorization Application (MAA) under exceptional circumstances for AAV-AIPL1 for the treatment of Leber congenital amaurosis (LCA4) retinal dystrophy without further clinical studies*
- *Announced positive data from randomized, sham-controlled clinical bridging study of AAV-GAD for the treatment of Parkinson's disease*

LONDON and NEW YORK, Nov. 13, 2024 (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, 2024, and provided a corporate update. The Company also announced that following meetings with the UK Medicines and Healthcare products Regulatory Agency (MHRA), the Company intends to submit a Marketing Authorization Application under exceptional circumstances for AAV-AIPL1 in the United Kingdom without the need for further clinical studies. The Company is also initiating discussions with the FDA around the potential for a similar pathway to approval in the U.S. In addition, MeiraGTx announced that the Offices of Orphan Products Development and Pediatric Therapeutics of the U.S. Food and Drug Administration (FDA) have granted the Company three Rare Pediatric Disease Designations to its AAV8-RK-AIPL1 program, AAV8-RK-BBS10 program, and AAV5-RDH12 program, each for the treatment of inherited retinal diseases.

"The past few months at MeiraGTx have been highlighted by exceptional clinical, regulatory, and research and development advancements," said Alexandria Forbes, Ph.D., president and chief executive officer of MeiraGTx. "In October, we announced positive data from our AAV-GAD bridging study using material manufactured in house at MeiraGTx. This study was a randomized, sham-controlled clinical study of AAV-GAD for the treatment of Parkinson's disease, demonstrating that the treatment is safe and leads to significant and clinically meaningful improvements in key efficacy endpoints including UPDRS Part 3 'off' score and PDQ-39 quality of life measure. Based on these extremely promising results, we are engaging with global regulatory agencies to initiate a Phase 3 registrational study."

Dr. Forbes added, "We also just received RPDD for three additional programs in our pipeline, a remarkable regulatory achievement for the Company. This underscores the groundbreaking therapeutic potential of our technology to uniquely address these severe childhood blinding conditions and offer hope to the families impacted. Upon FDA approval of a product with RPDD, we are eligible to receive a priority review voucher which could provide meaningful non-dilutive capital, as such vouchers have sold for $150 million and $158 million in recent weeks."

Dr. Forbes continued, "The AIPL1 program exemplifies how MeiraGTx has leveraged our internal manufacturing infrastructure and clinical expertise and worked with regulators to expedite by many years the delivery of these potentially life changing treatments to affected children. By releasing AAV-AIPL1 under our MHRA manufacturing specials license, MeiraGTx was uniquely placed to provide expert clinicians with a potential therapy for these children prior to formal clinical studies. LCA4 caused by mutations in the *AIPL1* gene results in blindness from birth, with complete degeneration of the retina by the age of four. Eleven children were treated between 1 and 4 years old with MeiraGTx's AAV-AIPL1 therapy. All 11 children, each of whom was blind from birth gained vision within 6 weeks of treatment. These extraordinary results supported a successful application to the MHRA Innovative Licensing and Access Pathway (ILAP), and with the award of the Innovation Passport, allowed an expedited Scientific Advice Meeting with the MHRA. During the meeting, agreement was reached that we are in a position to file a Marketing Authorization Application under exceptional circumstance based on the data from these 11 children with no further clinical studies required. In addition, because of our end-to-end internal manufacturing infrastructure, we have also agreed on the CMC requirement for approval. We have already engaged with the FDA to discuss a path to potential approval in the U.S., and we will continue to explore this type of expedited approval pathway for AIPL1 with other global agencies while investigating a similar strategy with the other RPDD awarded indications, including BBS10."

"The potential approval of transformative products for rare and devastating pediatric disorders in an expedited fashion is extremely exciting, allowing us to more rapidly advance potential treatments to severely impacted children many years faster than possible via the standard approval pathway. This is an illustration of the practical importance of optimizing our approach to viral vector development, as well as internalizing full commercial ready CMC capabilities, in developing effective treatments for rare, severe, rapidly degenerative diseases."

Recent Development Highlights and Anticipated Milestones

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

The primary study objective of safety and tolerability was met and significant and clinically meaningful improvements from baseline were demonstrated for key efficacy endpoints at 26 weeks.

Top-line data summary:

- AAV-GAD was safe and well tolerated, with no serious adverse events (SAEs) related to AAV-GAD treatment.
- At Week 26, a statistically significant 18-point average improvement from baseline in Unified Parkinson's Disease Rating

Scale (UPDRS) Part 3 “off” medication score was demonstrated in the high dose group (p=0.03), with no significant change in the sham or low dose groups. For the UPDRS Part 3 in the “off” state, a change of 5 to 10 points is considered clinically meaningful.

- Significant improvements from baseline in the disease-specific, patient-reported quality of life Parkinson’s Disease Questionnaire (PDQ-39) score were demonstrated in both the high and low dose groups with no significant change in the sham group at Week 26:
    - In the high dose AAV-GAD group, the PDQ-39 score improved by 8 points from baseline (p=0.02), the low dose group improved by 6 points from baseline (p=0.04), while the 0.2 point worsening in the sham surgery group was not statistically significant. For the PDQ-39, a 2 to 4-point change is considered clinically meaningful.
    - A dose response in PDQ-39 score was observed, with 100% of participants in the high dose group, 60% of participants in the low dose group, and 25% of participants in the sham surgery group reporting an improvement.
    - For the PDQ-39 score, there was a trend to significance between the high dose and sham surgery groups at 6 months (n=4 evaluable per group).

AAV-AIPL1 for the Treatment of Leber Congenital Amaurosis (LCA4) Retinal Dystrophy:

- Following recent meetings with the MHRA, the Company intends to submit a Marketing Authorization Application (MAA) under exceptional circumstances for AAV-AIPL1 in the United Kingdom.
- The Company is currently engaging with the FDA to discuss a path forward for regulatory approval in the United States.
- MeiraGTx was awarded an Innovation Passport designation by the U.K. Innovative Licensing and Access Pathway Steering Group for AAV8-RK-AIPL1.
- Meaningful responses have been observed in 11 out of 11 LCA4 children treated to date with AAV-AIPL1. All children were treated between 1 and 4 years old, all were blind on treatment, and all gained visual acuity 4 or more weeks following treatment.
- The Company’s AAV-AIPL1 for the treatment of inherited retinal dystrophy due to defects in the *AIPL1* gene has been granted orphan drug and now RPDD by the FDA and orphan designation by the European Commission.

Rare Pediatric Disease Designation Awards from the FDA:

- The Offices of Orphan Products Development and Pediatric Therapeutics of the FDA has granted RPDD to three of MeiraGTx’s inherited retinal disease programs:
    - AAV8-RK-AIPL1 for the treatment of LCA4 retinal dystrophy
    - AAV8-RK-BBS10 for the treatment of Bardet-Biedl syndrome (BBS) due to *BBS10* mutations
    - AAV5-RDH12 for the treatment of *RDH12* associated retinal dystrophy

An RPDD may be granted by the FDA to drugs and biologics intended to treat certain orphan diseases affecting fewer than 200,000 patients in the U.S., the serious or life-threatening manifestations of which primarily affect individuals aged 18 years or younger. Under the FDA’s Rare Pediatric Disease Priority Review Voucher (PRV) program, a sponsor that receives approval for a biologics license application for a rare pediatric disease may be eligible to receive a voucher for a priority review of a subsequent marketing application for a different product. PRVs may be used by the sponsor or sold to another sponsor for their use and have recently been sold for between $100 million to $158 million.

AAV2-hAQP1 for the Treatment of Xerostomia:

- Data from the Company’s Phase 1 AQUAx clinical trial were presented in an oral session at the American Academy of Oral Medicine (AAOM) 2024 annual meeting in April 2024, demonstrating that treatment with AAV2-hAQP1 resulted in significant improvements across three different patient-reported outcomes and in saliva production, with no treatment-related serious adverse events or dose-limiting toxicities reported.
- The Company continues to enroll and dose participants at multiple sites in the U.S., Canada and the U.K. in the Phase 2 AQUAx2 (NCT05926765) randomized, double-blind, placebo-controlled study.
- The Company recently gained alignment with the FDA on requirements for the ongoing Phase 2 AQUAx2 clinical trial for grade 2/3 radiation-induced xerostomia to be considered a pivotal trial in support of a potential BLA filing.

Botaretigene Sparoparvovec for the Treatment of XLRP:

- Data from the Phase 3 LUMEOS trial of botaretigene sparoparvovec (bota-vec) for the treatment of X-linked retinitis pigmentosa in collaboration with Johnson & Johnson Innovative Medicine is expected towards the end of this year. The Company is eligible to receive up to $285 million upon the first commercial sales of bota-vec in the U.S. 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.

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

- MeiraGTx continues to progress its riboswitch technology platform in multiple potential indications, with an initial focus on

obesity and metabolic disease and CAR-T for oncology and autoimmune disease.

- The Company continues to generate compelling preclinical data with metabolic peptides and hormones including incretins, myokines and leptin which suggests greater efficacy on weight loss as well as positive impact on fat to muscle ratio with certain novel combinations of peptides.
- The Company is in dialogue with regulatory agencies and intends to initiate first in human studies using the riboswitch platform for an undisclosed metabolic disease indication in 2025.

As of September 30, 2024, MeiraGTx had cash and cash equivalents of approximately $122.9 million as well as approximately $3.3 million in receivables due from Johnson & Johnson Innovative Medicine. The Company believes that with such funds, as well as anticipated near-term milestones from Johnson & Johnson Innovative Medicine under the asset purchase agreement, together with the tax incentive receivable, it will have sufficient capital to fund operating expenses and capital expenditure requirements into the second quarter of 2026. This estimate does not include 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, and for completion of the transfer of certain manufacturing technology.

Financial Results

Cash, cash equivalents and restricted cash were $125.0 million as of September 30, 2024, compared to $130.6 million as of December 31, 2023.

Service revenue was $10.9 million for the three months ended September 30, 2024 due to progress of process performance qualification (PPQ) services under the asset purchase agreement and related agreements with Johnson & Johnson Innovative Medicine.

There was no license revenue for the three months ended September 30, 2024, compared to $5.1 million for the three months ended September 30, 2023. The decrease is due to the termination of the collaboration agreement concurrent with the execution of the asset purchase agreement with Johnson & Johnson Innovative Medicine.

Cost of service revenue was $12.0 million for the three months ended September 30, 2024 due to progress of PPQ services under the asset purchase agreement and related agreements with Johnson & Johnson Innovative Medicine.

General and administrative expenses were $12.7 million for the three months ended September 30, 2024, compared to $10.0 million for the three months ended September 30, 2023. The increase of $2.7 million was primarily due to an increase in legal and accounting fees, other office related costs, payroll and payroll-related costs and consulting fees. These increases were partially offset by a decrease in share-based compensation, rent and facilities costs and insurance costs.

Research and development expenses for the three months ended September 30, 2024 were $26.2 million, compared to $27.9 million for the three months ended September 30, 2023. The decrease of $1.6 million was primarily due to a decrease in manufacturing costs primarily due to an increase in the number of batches of clinical trial material produced, which costs were charged to the clinical programs, a reduction in manufacturing material purchases during the three months ended September 30, 2024 compared to the three months ended September 30, 2023 as well as a reclassification of cost of service revenue due to progress of PPQ services provided under the asset purchase agreement and related agreements. This decrease was partially offset by a reduction in reimbursements from Johnson & Johnson Innovative Medicine as the reimbursement for the three months ended September 30, 2023 was in connection with research funding provided under the collaboration agreement, which was terminated on December 20, 2023. Expenses related to our preclinical programs increased primarily related to development of our preclinical ocular disease programs and clinical trial expenses increased primarily due to an increase in the number of batches of clinical trial material produced during the three months ended September 30, 2024 compared to the three months ended September 30, 2023, which costs were charged from manufacturing costs to the clinical programs. The increase in clinical trial expenses was partially offset by a decrease in costs related to bota-vec as Johnson & Johnson Innovative Medicine is now primarily funding the expenses related to this program as a result of the asset purchase agreement. Additionally, other research and development expenses increased.

Foreign currency gain was $3.5 million for the three months ended September 30, 2024, compared to a loss of $8.7 million for the three months ended September 30, 2023. The change of $12.1 million was primarily due to the restructuring and payment of certain intercompany receivables and payables. Foreign currency gains and losses subsequent to the restructuring are recorded as a part of accumulated other comprehensive income.

Interest income was $1.2 million for the three months ended September 30, 2024, compared to $0.5 million for the three months ended September 30, 2023. The increase of $0.7 million was due to higher interest rates and cash balances during 2024.

Interest expense was $3.4 million for each of the three months ended September 30, 2024 and September 30, 2023.

Loss on sale of nonfinancial assets was $0.6 million for the three months ended September 30, 2024, which was a result of an adjustment to the allocation of the transaction price and $50.0 million milestone payment to the performance obligations identified under the asset purchase agreement. The nonfinancial assets were sold and assigned to Johnson & Johnson Innovative Medicine including a License Agreement between the Company and UCL Business Plc (now UCL Business Ltd.) relating to the research, development, manufacture and exploitation of bota-vec, and other related assets pursuant in the asset purchase agreement.

Net loss attributable to ordinary shareholders for the quarter ended September 30, 2024, was $39.3 million, or $0.55 basic and diluted net loss per ordinary share, compared to a net loss attributable to ordinary shareholders of $44.3 million, or $0.74 basic and diluted net loss per ordinary share for the quarter ended September 30, 2023.

About AAV8-RK-AIPL1

AAV8-RK-AIPL1 is an investigational genetic medicine for the treatment of one of the most severe forms of Leber congenital amaurosis (LCA) owing to genetic deficiency of Aryl-hydrocarbon-interacting protein-like 1 (AIPL1). It is delivered via subretinal injection to children, and through a one-time administration, AAV8-RK-AIPL1 is designed to deliver functional copies of the AIPL1 gene to cone and rod photoreceptors in the central retina to slow further degeneration and restore vision.

About AAV8-RK-BBS10

The investigational genetic medicine AAV8-RK-BBS10 is an adeno-associated virus with a serotype 8 capsid with a complementary DNA (cDNA) encoding the human *BBS10* gene for treatment of Bardet-Biedl syndrome (BBS) due to *BBS10* mutations. BBS is a rare genetic disease affecting approximately 1 in 250,000 people around the world. One of the primary symptoms of BBS is visual impairment secondary to retinal degeneration. More than 20 different genes are associated with the development of BBS, with BBS10 accounting for approximately 25% of cases.

About AAV5-RDH12

The investigational genetic medicine AAV5-RDH12 is an adeno-associated virus serotype 5 containing the human *RDH12* gene for treatment of *RDH12* associated retinal dystrophy. Defects in retinol dehydrogenase 12 (*RDH12*) account for 3–10% of Leber congenital amaurosis (LCA) and early-onset severe retinal dystrophy (EOSRD) and is particularly devastating due to early macular atrophy. *RDH12* encodes retinol dehydrogenase 12, an enzyme expressed in photoreceptors that reduces all-trans-retinal to all-*trans*-retinol.

About MeiraGTx

MeiraGTx (Nasdaq: MGTX) is a vertically integrated, clinical-stage genetic medicines company with a broad pipeline of late-stage clinical programs supported by end-to-end manufacturing capabilities. MeiraGTx has internal plasmid production for GMP, two GMP viral vector production facilities as well as an in-house Quality Control hub for stability and release, all fit for IND through commercial supply. In addition, MeiraGTx has developed a proprietary manufacturing platform with leading yield and quality aspects and commercial readiness, core capabilities in viral vector design and optimization and a transformative riboswitch gene regulation platform technology that allows for the precise, dose-responsive control of gene expression by oral small molecules. MeiraGTx is focusing the riboswitch platform on the delivery of metabolic peptides, including GLP-1, GIP, Glucagon, and PYY, using oral small molecules, as well as cell therapy for oncology and autoimmune diseases. MeiraGTx has developed the technology to apply genetic medicine to more common diseases, increasing efficacy, addressing novel targets, and expanding access in some of the largest disease areas where the unmet need remains high.

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, as well as statements that include the words “expect,” “will,” “intend,” “plan,” “believe,” “project,” “forecast,” “estimate,” “may,” “could,” “should,” “would,” “continue,” “anticipate” and similar statements of a future or forward-looking nature. These forward-looking statements are based on management’s current expectations. These statements are neither promises nor guarantees, but involve known and unknown risks, uncertainties and other important factors that may cause actual results, performance or achievements to be materially different from any future results, performance or achievements expressed or implied by the forward-looking statements, including, but not limited to, 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, 2024, 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)

	ForttheThree-Month Periods Ended September 30,		FortheNine-Month Periods Ended September 30,	
	2024	2023	2024	2023
Revenues:				
Service revenue – related party	$ 10,910	$ —	$ 11,889	$ —
License revenue – related party	—	5,103	—	11,977
Total revenue	10,910	5,103	11,889	11,977
Operating expenses:				
Cost of service revenue – related party	11,985	—	11,985	—
General and administrative	12,723	10,009	37,127	35,169
Research and development	26,243	27,856	95,499	70,115
Total operating expenses	50,951	37,865	144,611	105,284
Loss from operations	(40,041)	(32,762)	(132,722)	(93,307)
Other non-operating income (expense):				
Foreign currency gain (loss)	3,463	(8,677)	2,644	(2,915)
Interest income	1,189	523	3,113	1,723
Interest expense	(3,357)	(3,381)	(9,861)	(9,796)
(Loss) gain on sale of nonfinancial assets	(584)	—	28,434	—
Fair value adjustment	—	—	—	53
Net loss	(39,330)	(44,297)	(108,392)	(104,242)
Other comprehensive loss:				
Foreign currency translation (loss) gain	(1,234)	6,007	(3,413)	1,113
Comprehensive loss	$ (40,564)	$ (38,290)	$ (111,805)	$ (103,129)
Net loss	$ (39,330)	$ (44,297)	$ (108,392)	$ (104,242)
Basic and diluted net loss per ordinary share	$ (0.55)	$ (0.74)	$ (1.62)	$ (1.91)
Weighted-average number of ordinary shares outstanding	71,633,150	59,526,642	66,709,847	54,544,660

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

	September30, 2024	December31, 2023
ASSETS		
CURRENT ASSETS:		
Cash and cash equivalents	$ 122,873	$ 129,566
Accounts receivable – related party	3,279	10,138
Prepaid expenses	7,029	5,625
Tax incentive receivable	5,152	13,277
Other current assets	713	1,016
Total Current Assets	139,046	159,622
Property, plant and equipment, net	112,541	115,896
Intangible assets, net	951	1,118
Restricted cash	2,156	1,083
Other assets	1,139	1,917
Equity method and other investments	6,766	6,766
Right-of-use assets – operating leases, net	12,782	15,910
Right-of-use assets – finance leases, net	24,107	24,432
TOTAL ASSETS	$ 299,488	$ 326,744
LIABILITIES AND SHAREHOLDERS' EQUITY		
CURRENT LIABILITIES:		

Accounts payable	$ 29,504	$ 16,042
Accrued expenses	19,341	42,639
Lease obligations, current	4,183	4,193
Deferred revenue – related party, current	5,107	2,926
Other current liabilities	1,283	1,278
Total Current Liabilities	59,418	67,078
Deferred revenue – related party	58,902	34,017
Lease obligations	9,610	12,952
Asset retirement obligations	2,880	2,401
Note payable, net	72,942	72,119
TOTAL LIABILITIES	203,752	188,567
COMMITMENTS AND CONTINGENCIES (Note 11)		
SHAREHOLDERS' EQUITY:		
Ordinary Shares, $0.00003881 par value, 1,288,327,750 authorized, 77,695,418 and 63,601,015 shares issued and outstanding at September 30, 2024 and December 31, 2023, respectively	3	2
Capital in excess of par value	763,204	693,841
Accumulated other comprehensive loss	(4,848)	(1,435)
Accumulated deficit	(662,623)	(554,231)
Total Shareholders' Equity	95,736	138,177
TOTAL LIABILITIES AND SHAREHOLDERS' EQUITY	$ 299,488	$ 326,744