MeiraGTx Reports First Quarter 2024 Financial and Operational Results

May 09, 2024

- Positive data from the Phase 1 AQUAx study in radiation-induced xerostomia (RIX) presented in an oral session at the American Academy of Oral Medicine 2024 annual meeting (AAOM) April 17-20, 2024

- Received $50 million milestone following initiation of the extension study for the Phase 3 LUMEOS clinical trial for botaretigene sparoparvovec (bota-vec, formerly AAV-RPGR) for the treatment of X-linked retinitis pigmentosa (XLRP)

- Clinical development milestones met and remain on track for the rest of the year for AAV-hAQP1 for RIX, bota-vec for XLRP, and AAV-GAD for Parkinson's disease

LONDON and NEW YORK, May 09, 2024 (GLOBE NEWSWIRE) -- MeiraGTx Holdings plc (Nasdaq: MGTX), a vertically integrated, clinical stage gene therapy company, today announced financial and operational results for the first quarter ended March 31, 2024, and provided a corporate update.

"MeiraGTx's first quarter was highlighted by significant progress in our wholly-owned late stage clinical programs, including our pivotal xerostomia program and Parkinson's program as well as our riboswitch platform," said Alexandria Forbes, Ph.D., president and chief executive officer of MeiraGTx. "At this year's AAOM meeting in April, extremely encouraging data was presented from the Phase 1 AQUAx study, showing that treatment with AAV2-hAPQ1 resulted in significant improvements in 3 different patient reported outcomes (PROs) as well as unprecedented improvement in the production of saliva. We continue to hear from investigators that our therapy is 'life changing' for their patients fortunate enough to have had access to the treatment. Dosing in our pivotal Phase 2 AQUAx2 trial is ongoing, and we are pleased to have aligned with FDA on requirements for this Phase 2 study to be considered pivotal to support potential BLA filing for this significant unmet need."

Dr. Forbes continued: "Having completed dosing in our bridging study for AAV-GAD manufactured at MeiraGTx, we remain on track to discuss Phase 3 study design with global regulators for Parkinson's disease in the second half of 2024. In addition, we continue to anticipate data from the large, multi-center Phase 3 study of bota-vec for XLRP-RPGR in collaboration with Janssen Pharmaceuticals, Inc. (Janssen) towards the end of this year, setting up the potential $285 million in milestones under the asset purchase agreement we entered into with Janssen late last year."

"We are particularly excited by the recent data we have seen with our riboswitch platform for *in vivo* delivery of short acting metabolic peptides," said Dr. Forbes. "The data in metabolic disease models are striking and the approach highly differentiated. By precisely controlling the timing and levels of production of native forms of short acting peptides in the body, we demonstrate significant increase in efficacy versus the same persistently active peptide combinations. This degree of improvement in efficacy is consistent with the data we have demonstrated with CAR-T, where precise periodic control of CAR levels transforms the CAR-T cell profile to that of naïve T-cells, prevents exhaustion and increases efficacy *in vivo* by three to four fold over CAR-T cells containing unregulated CAR."

"Our technology also allows the *in vivo* production of novel peptides, for example those that directly regulate muscle mass and fat accumulation. The ability to deliver any combination of peptides in a controlled physiological timeframe with oral small molecules has the potential to address many of the issues that arise with current therapies for obesity and diabetes, including: significantly enhanced efficacy, improved tolerability, improved muscle mass and prevention of the regain of fat. Importantly, our technology also decreases the manufacturing burden for peptides since the body produces the peptides *in vivo* in response to oral small molecules."

Dr. Forbes continued, "We are prioritizing moving one or more of these incretin, myokine and adipokine combinations towards the clinic to provide a completely differentiated approach to addressing obesity and metabolic disease."

Recent Development Highlights and Anticipated Milestones

Phase 1 AQUAx Study of AAV-hAQP1 for the Treatment of Grade 2/3 Radiation-Induced Xerostomia:
Grade 2/3 radiation-induced xerostomia (RIX) is a severely debilitating consequence of radiation treatment for head and neck cancer that affects approximately 30-40% of all patients treated with radiation for head and neck cancer. This is a completely unmet need with no treatment options, and a large addressable market with over 170,000 patients currently in the U.S., and an additional 15,000 new patients in the U.S. each year. Treatment with AAV-hAQP1 involves a small dose locally delivered to the salivary gland via a non-invasive procedure, that can be delivered in a dental office or oncology center where these patients are seen at least annually following radiation treatment. The small local dose of AAV-hAQP1 manufactured in-house at MeiraGTx allows for a low cost of goods, and the potential long-term durability and ease of delivery make this large addressable market a compelling commercial opportunity. In addition to the RIX patient population from treatment for head and neck cancer, new therapies such as PSMA-targeted radioligand therapy can also lead to xerostomia, providing additional potential patient populations that may benefit from our AAV-hAQP1 treatment.

An oral presentation of the Phase 1 AQUAx study, entitled "Results of a Phase 1, Open-label, Dose-escalation Study of Gene Therapy with AAV2-hAQP1 as Treatment for Grade 2 and 3 Radiation-induced Late Xerostomia and Parotid Gland Hypofunction – The AQUAx Study" was delivered at the Academy of Oral Medicine 2024 annual meeting that took place April 17-20, 2024.

Summary of Findings presented at AAOM:

- No treatment-related serious adverse events or dose-limiting toxicities were reported, and all participants completed the study.

- The 3 different PRO instruments showed statistically significant improvements by Day 30 that were maintained through Month 12:
    - At Month 12, the average Total XQ Score improved by 17 points (39.5%) from baseline and 16 of 24 participants reported an improvement of ≥8 points
    - At Month 12, the MDASI-HN-DM score improved by 2.7 points (42.2%) from baseline
    - At Month 12, the average improvement in GRCQ Score was 3.8
    - Across the PROs, bilaterally-treated participants reported greater improvement than those treated unilaterally
- At Month 12, the Unstimulated Whole Saliva Flow Rate increased from baseline by 112.8%

Phase 2 AQUAx2 Study:

- The Company continues to enroll and dose participants at multiple sites in the U.S. and Canada in the Phase 2 AQUAx2 randomized, double-blind, placebo-controlled study.
- The Company aligned with the FDA on requirements for the ongoing Phase 2 AQUAx2 clinical trial for Grade 2/3 radiation-induced xerostomia to be considered pivotal to support potential BLA filing.
- Received Clinical Trial Authorization approval from the UK Medicines and Healthcare products Regulatory Agency (MHRA) for AQUAx2 study in the UK. Site activations are ongoing.

AAV-GAD for the Treatment of Parkinson's Disease :
Parkinson's is the second most common neurodegenerative disease after Alzheimer's with approximately 90,000 patients diagnosed annually in the U.S. Most Parkinson's patients respond to dopamine replacement therapy, however, after about 5 years, even higher doses of dopamine no longer manage the motor symptoms, leaving little effective treatment for this large population of patients. MeiraGTx's gene therapy treatment for Parkinson's involves the delivery of a very small dose of AAV-GAD encoding the enzyme that converts the activating neurotransmitter glutamate to the calming neurotransmitter GABA, to the specific nucleus of the brain targeted that blocks signaling to the motor cortex. We have demonstrated that localized treatment with AAV-GAD leads to a change in circuitry to the motor cortex, resulting in alleviation of motor symptoms. This is a one-time treatment with a small dose of viral vector that has a low cost of goods addressing a significant need in a large patient population.

- The Company completed dosing patients in the Phase 1 trial of AAV-GAD under a new IND with material manufactured in its GMP facility in London, United Kingdom using MeiraGTx's proprietary production process.
- The AAV-GAD trial is a three-arm randomized Phase 1 clinical bridging study with subjects randomized to sham control or one of two doses of AAV-GAD.
- The objective of the AAV-GAD trial (NCT05603312) is to evaluate the safety and tolerability of AAV-GAD when delivered to the subthalamic nucleus (STN) of patients with Parkinson's disease.
- The Company intends to initiate Phase 3 study design discussions with global regulatory agencies in the second half of 2024.

Riboswitch Gene Regulation Technology Platform:
MeiraGTx's riboswitch technology allows for repeatable, long-term delivery of any messenger RNA (mRNA) from the DNA template encoding any peptide or protein on activation by bespoke orally delivered small molecules.

- For obesity and metabolic disease, the Company has successfully delivered multiple combinations of gut peptides *in vivo* including GLP-1, GIP, PYY, Glucagon, and Oxyntomodulin as well as novel myokine and adipokine peptides that drive muscle metabolism and fat storage, via the riboswitch platform. The technology allows daily dosing with a small molecule to drive production of peptides within the body in physiologically relevant combinations and timing. This provides a platform for addressing not just weight loss via reduced appetite, but also muscle strength, fat metabolism, and cardiovascular health and neurodegenerative disorders in metabolic disease, with daily oral small molecules.
- In CAR-T for both oncology and autoimmune disease, precise control of levels and timing of the CAR has demonstrated a significant impact on CAR-T efficacy, with a 3-4 fold improvement in *in vivo* potency of T-cells with regulated CAR compared to the currently approved CAR-T with unregulated constitutively active CAR. In addition, MeiraGTx's regulated CAR-T display a normal naïve T-cell profile, lacking exhaustion markers and retaining proliferation and killing ability in contrast to CAR-T with unregulated constitutive CAR expression.
- The Company intends to present data from its riboswitch gene regulation technology platform at an R&D Day in the second half of 2024.

Bota-vec for the Treatment of XLRP:

- In March, the Company received a $50 million milestone after initiation of the extension study for the Phase 3 LUMEOS clinical trial for bota-vec for the treatment of XLRP.
- MeiraGTx anticipates receiving an additional $15 million in near-term milestone payments later in 2024.
- The Company will receive up to a further $285 million upon first commercial sales of bota-vec in the U.S. and EU and for manufacturing technology transfer.
- MeiraGTx also entered into a commercial supply agreement with Janssen for bota-vec manufacturing, which the Company anticipates will generate additional revenue during the product launch.

AAV-AIPL1 Specials License in the UK:

- Meaningful responses have been observed in 8 out of 8 LCA4 children treated to date with AAV-AIPL1. All children were treated between 1 and 3 years old, all were blind on treatment, and all gained visual acuity 4 or more weeks following treatment.
- Given the positive results under the Specials License, MeiraGTx has engaged with regulatory agencies in the UK to enable the Company to make this intervention more widely available to the LCA4 patient population globally.
- The Company's AAV-AIPL1 for the treatment of inherited retinal dystrophy due to defects in the AIPL1 gene has been granted orphan drug designation by the FDA and orphan designation by the European Commission and the Company anticipates receiving a Rare Pediatric Disease Designation (RPDD).

As of March 31, 2024, MeiraGTx had cash and cash equivalents of approximately $119.2 million and in April 2024, the Company collected $19.7 million in receivables which were due in the first quarter of 2024, effectively increasing the cash balance to $138.9 million. The payments consisted of a $9.6 million refund in connection with a research and development tax credit and $10.1 million from Janssen in connection with the collaboration agreement. The Company believes that with such funds, as well as anticipated near-term milestones and receivables from Janssen under the asset purchase agreement, it will have sufficient capital to fund operating expenses and capital expenditure requirements into the first 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, for completion of the transfer of certain manufacturing technology.

Financial Results

Cash, cash equivalents and restricted cash were $120.3 million as of March 31, 2024, compared to $130.6 million as of December 31, 2023.

Service revenue was $0.7 million for the three months ended March 31, 2024 due to progress of process performance qualification services under the asset purchase agreement with Janssen.

There was no license revenue for the three months ended March 31, 2024, compared to $3.3 million for the three months ended March 31, 2023. The decrease is due to the termination of the collaboration agreement concurrent with the execution of the asset purchase agreement with Janssen.

General and administrative expenses were $13.1 million for the three months ended March 31, 2024, compared to $12.8 million for the three months ended March 31, 2023. The increase of $0.4 million was primarily due to an increase in share-based compensation, payroll and payroll-related costs and other office related costs. These increases were partially offset by a decrease in legal and accounting fees and insurance costs.

Research and development expenses for the three months ended March 31, 2024, were $34.3 million, compared to $22.3 million for the three months ended March 31, 2023. The increase of $12.0 million was primarily due to an increase in manufacturing costs primarily due to a decrease in the number of batches of clinical trial material produced during the three months ended March 31, 2024 compared to the three months ended March 31, 2023 which were charged to the clinical programs and a decrease in Janssen reimbursements as the reimbursement for the three months ended March 31, 2023 was in connection with research funding provided under the collaboration agreement which was terminated on December 20, 2023, whereas the reimbursement for the three months ended March 31, 2024 was in connection with transition services the Company provided to Janssen. These increases were partially offset by decreases in clinical trial expenses primarily related to bota-vec as a result of a decrease in the number of batches of clinical trial material produced in the three months ended March 31, 2024 compared to the three months ended March 31, 2023 and in addition, as a result of the asset purchase agreement, Janssen is now primarily funding the expenses related to this program. Additionally, other research and development costs decreased as well as expenses related to the Company's preclinical programs, primarily related to preclinical ocular diseases.

Foreign currency loss was $0.5 million for the three months ended March 31, 2024, compared to a gain of $3.9 million for the three months ended March 31, 2023. The change of $4.4 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 $1.1 million for the three months ended March 31, 2024, compared to $0.5 million for the three months ended March 31, 2023. The increase of $0.6 million was due to higher interest rates and cash balances during 2024.

Interest expense was $3.3 million for the three months ended March 31, 2024, compared to $3.1 million for the three months ended March 31, 2023. The increase of $0.2 million was primarily due to a higher interest rate in connection with the debt financing.

Gain on sale of nonfinancial assets was $29.0 million for the three months ended March 31, 2024 compared to $0 for the three months ended March 31, 2023. This increase was a result of the recognition of the $50.0 million milestone allocated to the nonfinancial assets sold and assigned to Janssen 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 to the asset purchase agreement.

Net loss attributable to ordinary shareholders for the quarter ended March 31, 2024, was $20.4 million, or $0.32 basic and diluted net loss per ordinary share, compared to a net loss attributable to ordinary shareholders of $30.4 million, or $0.62 basic and diluted net loss per ordinary share for the quarter ended March 31, 2023.

About MeiraGTx

MeiraGTx (Nasdaq: MGTX) is a vertically integrated, clinical-stage gene therapy company with a broad pipeline of late-stage clinical programs supported by end-to-end manufacturing capabilities. MeiraGTx has an internally developed manufacturing platform process, 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. MeiraGTx has core capabilities in viral vector design and optimization and a potentially 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 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. Although initially focusing on the eye, central nervous system, and salivary gland, 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 great.

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, our ability to manufacture product candidates, potential milestone payments and the achievement of such milestones, including the receipt of such milestone payments and the impact on our cash runway, and 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 designations, retain key personnel or attract qualified employees, or incur expected levels of operating expenses; the impact of pandemics, epidemics or outbreaks of infectious diseases on the status, enrollment, timing and results of our clinical trials and on our business, results of operations and financial condition; failure of early data to predict eventual outcomes; failure to obtain FDA or other regulatory approval for product candidates within expected time frames or at all; the novel nature and impact of negative public opinion of gene therapy; failure to comply with ongoing regulatory obligations; contamination or shortage of raw materials or other manufacturing issues; changes in healthcare laws; risks associated with our international operations; significant competition in the pharmaceutical and biotechnology industries; dependence on third parties; risks related to intellectual property; changes in tax policy or treatment; our ability to utilize our loss and tax credit carryforwards; litigation risks; and the other important factors discussed under the caption “Risk Factors” in our Quarterly Report on Form 10-Q for the quarter ended March 31, 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)

	For the Three-Month Periods Ended March 31,	
	2024	2023
Revenues:		
Service revenue - related party	$ 697	$ —
License revenue - related party	—	3,334
Total revenue	697	3,334
Operating expenses:		
General and administrative	13,147	12,772
Research and development	34,322	22,322
Total operating expenses	47,469	35,094
Loss from operations	(46,772)	(31,760)
Other non-operating income (expense):		
Foreign currency (loss) gain	(535)	3,857
Interest income	1,097	545

Interest expense	(3,250)	(3,060)
Gain on sale of nonfinancial assets	29,018	—
Fair value adjustment	—	54
Net loss	(20,442)	(30,364)
Other comprehensive loss:		
Foreign currency translation loss	(1,691)	(2,353)
Comprehensive loss	$ (22,133)	$ (32,717)
Net loss	$ (20,442)	$ (30,364)
Basic and diluted net loss per ordinary share	$ (0.32)	$ (0.62)
Weighted-average number of ordinary shares outstanding	64,065,895	48,638,151

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

	March 31, 2024	December 31, 2023
ASSETS		
CURRENT ASSETS:		
Cash and cash equivalents	$ 119,206	$ 129,566
Accounts receivable - related party	10,915	10,138
Prepaid expenses	5,076	5,625
Tax incentive receivable	13,171	13,277
Other current assets	932	1,016
Total Current Assets	149,300	159,622
Property, plant and equipment, net	111,412	115,896
Intangible assets, net	1,038	1,118
Restricted cash	1,059	1,083
Other assets	1,138	1,917
Equity method and other investments	6,766	6,766
Right-of-use assets - operating leases, net	14,835	15,910
Right-of-use assets - finance leases, net	23,687	24,432
TOTAL ASSETS	$ 309,235	$ 326,744
LIABILITIES AND SHAREHOLDERS' EQUITY		
CURRENT LIABILITIES:		
Accounts payable	$ 21,223	$ 16,042
Accrued expenses	17,353	42,639
Lease obligations, current	4,188	4,193
Deferred revenue - related party, current	3,772	2,926
Other current liabilities	1,007	1,278
Total Current Liabilities	47,543	67,078
Deferred revenue - related party	53,331	34,017
Lease obligations	11,796	12,952
Asset retirement obligations	2,440	2,401
Note payable, net	72,391	72,119
TOTAL LIABILITIES	187,501	188,567
COMMITMENTS AND CONTINGENCIES (Note 11)		
SHAREHOLDERS' EQUITY:		
Ordinary Shares, $0.00003881 par value, 1,288,327,750 authorized, 64,298,691 and 63,601,015 shares issued and outstanding at March 31, 2024 and December 31, 2023, respectively	2	2
Capital in excess of par value	699,531	693,841
Accumulated other comprehensive loss	(3,126)	(1,435)
Accumulated deficit	(574,673)	(554,231)
Total Shareholders' Equity	121,734	138,177
TOTAL LIABILITIES AND SHAREHOLDERS' EQUITY	$ 309,235	$ 326,744