MeiraGTx Reports First Quarter 2023 Financial and Operational Results

May 11, 2023

- *Raised approximately $60 million in a private placement in May 2023 with investors consisting of several of the Company's top shareholders*
- *On track for BLA submission of botaretigene sparoparvovec (bota-vec, formerly AAV-RPGR) for the treatment of X-linked retinitis pigmentosa (XLRP) in 2024*
- *Company will present 12-month data from bilateral treated cohorts from the AQUAx AAV-hAQP1 Phase 1 study for treatment of Grade 2/3 radiation-induced xerostomia in the second quarter of 2023*
- *Upcoming poster presentations at the American Society of Gene and Cell Therapy (ASGCT) 2023 Annual Meeting highlight the depth and novelty of MeiraGTx's technology platforms for gene and cell therapy*

LONDON and NEW YORK, May 11, 2023 (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, 2023, and provided a corporate update.

"We are off to a strong start this year with progress across multiple clinical-stage programs and a growing body of data supporting our proprietary gene regulation technology," said Alexandria Forbes, Ph.D., president and chief executive officer of MeiraGTx. "Enrollment of the pivotal Lumeos Phase 3 study is going well and we remain on track for a BLA filing in 2024. We are also excited to be progressing towards later stage studies in our wholly-owned programs, including a randomized, double-blind, placebo-controlled, Phase 2 study for the treatment of Grade 2/3 radiation-induced xerostomia, and our randomized, sham-controlled bridging study for our Parkinson's disease program."

Dr. Forbes continued, "At this year's ARVO Annual Meeting, we presented data from our AI-driven promoter discovery platform illustrating our ability to precisely target specific cells and expression levels with synthetic promoters. In addition we presented immune-response data from the Phase 1/2 trial for the investigational gene therapy bota-vec in patients with XLRP associated with mutations in the *RPGR* gene. We are also pleased that nine poster presentations showcasing our novel gene and cell therapy platforms will be presented at the upcoming ASGCT Annual Meeting, including the application of our riboswitch technology to precisely regulate CAR-Ts. We are also presenting data demonstrating efficacy in animal models of vector delivered human growth hormone and anti-HER2 antibody in tight dose response to an orally dosed small molecule inducer. This provides support for the broad applicability of our riboswitch gene regulation platform for therapeutic delivery of a range of biologic drugs and cell therapies with enhanced safety and efficacy."

Recent Development Highlights and Anticipated Milestones

Bota-vec for the Treatment of XLRP:

- In late April, immune-response data from a Phase 1/2 MGT009 clinical trial (NCT03252847) were presented at the Association for Research in Vision and Ophthalmology (ARVO) 2023 Annual Meeting.
- Dosing in the pivotal Phase 3 LUMEOS clinical trial of bota-vec continues and the program remains on track for a BLA submission in 2024.

AAV-hAQP1 for the Treatment of Grade 2/3 Radiation-Induced Xerostomia:

- MeiraGTx reported positive clinical data from the AQUAx Phase 1 clinical trial in December 2022, and remains on track to present the full data from the AQUAx Phase 1 study in the second quarter of 2023, including the 12 month data from bilaterally treated subjects.
- Based on the favorable safety and efficacy profile of AAV-hAQP1 in the AQUAx Phase 1 study, the Company intends to initiate a randomized, double-blind, placebo-controlled, Phase 2 study evaluating the bilateral administration of two active doses of AAV-hAQP1 in the second quarter of 2023.

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

- The Company is now dosing patients in the AAV-GAD clinical trial under a new IND with material manufactured in its cGMP 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 manufactured at

MeiraGTx's cGMP facility in London, United Kingdom when delivered to the subthalamic nucleus (STN) of patients with Parkinson's disease.
- Completion of enrollment is anticipated in the third quarter of 2023.

Riboswitch Gene Regulation Platform & Vector Engineering:

- The Company will exhibit nine poster presentations at the ASGCT 2023 Annual Meeting, including data demonstrating the ability to regulate CAR-T driving increased efficacy and reduced exhaustion *in vitro*.
- ASGCT posters will also show data demonstrating rescue of B.little mouse model via hGH activated using an orally delivered small molecule, as well as AAV-mediated riboswitch-controlled delivery of anti-HER2 antibody suppressing HER2-positive tumorigenesis in a dose response to an orally delivered small molecule.
- The Company's next-generation riboswitch-based gene regulation platform can be used to precisely control the expression of any gene delivered in any context with an unprecedented dynamic range using novel, synthetic, orally delivered small molecules.
- The Company now has over 40 novel orally available small molecules with high specificity and potency to its riboswitch aptamers moving through PK, biodistribution, and toxicology studies, with the first GMP material for IND currently being manufactured. Two of these small molecules show good brain penetrance to enable activation of genes within the blood brain barrier.

Recent Corporate Developments
In May 2023, MeiraGTx closed the previously announced private investment in public equity (PIPE) financing, raising approximately $60 million in aggregate gross proceeds. The financing was entirely led by several of the Company's top holders Perceptive Advisors, Adage Capital, Prosight Management, and 683 Capital Management.

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

In addition to the proceeds from the PIPE financing, MeiraGTx had cash and cash equivalents of approximately $68.8 million, as well as approximately $36.3 million in receivables due from Janssen. The Company believes that with such funds, as well as anticipated milestones from Janssen, it will have sufficient capital to fund operating expenses and capital expenditure requirements into the second quarter of 2025.

Financial Results

Cash and cash equivalents were $68.8 million as of March 31, 2023, compared to $115.5 million as of December 31, 2022.

License revenue was $3.3 million for the quarter ended March 31, 2023, compared to $5.6 million for the quarter ended March 31, 2022. This decrease represents decreased amortization of the $100.0 million upfront payment as well as decreased amortization of the $30.0 million milestone payment received in connection with the Janssen collaboration.

General and administrative expenses were $12.8 million for the three months ended March 31, 2023, compared to $11.3 million for the three months ended March 31, 2022. The increase of $1.5 million was primarily due to an increase in legal and accounting fees, consulting fees and other office related costs. These increases were partially offset by decreases in share-based compensation, insurance costs and payroll and payroll-related costs.

Research and development expenses for the three months ended March 31, 2023 were $22.3 million, compared to $23.1 million for the three months ended March 31, 2022. The decrease of $0.8 million was primarily due to a decrease in expenses related to our preclinical programs primarily due to the timing of expenses in our gene regulation program and a decrease in other research and development expenses primarily due to a decrease in share-based compensation, as well as an increase in research funding provided under our Janssen collaboration primarily due to the increase in expenses incurred related to our program for bota-vec for the treatment of XLRP. These decreases were partially offset by an increase in clinical trial expenses primarily due to an increase in expenses related to our Phase 3 Lumeos clinical trial of bota-vec and our expanded Phase 1 clinical trial and Phase 2 clinical trial for AAV-hAQP1, as well as an increase in manufacturing expenses primarily due to the commencement of operations at our Shannon, Ireland manufacturing facility in 2022.

Foreign currency gain was $3.9 million for the three months ended March 31, 2023, compared to a loss of $2.6 million for the three months ended March 31, 2022. The change of $6.5 million was primarily due to an unrealized gain on the quarterly valuation of intercompany payables and receivables due to the weakening of the U.S. dollar against the pound sterling and euro during the three months ended March 31, 2023.

Net loss attributable to ordinary shareholders for the quarter ended March 31, 2023 was $30.4 million, or $0.62 basic and diluted net loss per ordinary share, compared to a net loss attributable to ordinary shareholders of $31.0 million, or $0.70 basic and diluted net loss per ordinary share for the quarter ended March 31, 2022.

About MeiraGTx
MeiraGTx (Nasdaq: MGTX) is a vertically integrated, clinical-stage gene therapy company with six programs in clinical development and a broad pipeline of preclinical and research programs. MeiraGTx has core capabilities in viral vector design and optimization and gene therapy manufacturing, and a transformative gene regulation platform technology that allows precise, dose responsive control of gene expression by oral small molecules with dynamic range that can exceed 5000-fold. Led by an experienced management team, MeiraGTx has taken a portfolio approach by licensing, acquiring, and developing technologies that give depth across both product candidates and indications. MeiraGTx's initial focus is on three distinct areas of unmet medical need: ocular diseases, including both inherited retinal diseases as well as large degenerative ocular diseases, neurodegenerative diseases and severe forms of xerostomia. Though initially focusing on the eye, central nervous system, and salivary gland, MeiraGTx plans to expand its focus to develop additional gene therapy treatments for patients suffering from a range of serious diseases.

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 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 the COVID-19 pandemic 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, 2023, 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.

Contacts

Investors:
MeiraGTx
Investors@meiragtx.com

or

Media:
Jason Braco, Ph.D.
LifeSci Communications
jbraco@lifescicomms.com

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 Period Ended March 31,	
	2023	2022
License revenue - related party	$ 3,334	$ 5,633
Operating expenses:		
General and administrative	12,772	11,268
Research and development	22,322	23,099
Total operating expenses	35,094	34,367
Loss from operations	(31,760)	(28,734)
Other non-operating income (expense):		
Foreign currency gain (loss)	3,857	(2,647)
Interest income	545	16
Interest expense	(3,060)	(77)
Fair value adjustment	54	397
Net loss	(30,364)	(31,045)
Other comprehensive (loss) income:		
Foreign currency translation (loss) gain	(2,353)	1,932
Comprehensive loss	$ (32,717)	$ (29,113)
Net loss	$ (30,364)	$ (31,045)
Basic and diluted net loss per ordinary share	$ (0.62)	$ (0.70)

Weighted-average number of ordinary shares outstanding	48,638,151	44,501,314

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

	March31, 2023	December31, 2022
ASSETS		
CURRENT ASSETS:		
Cash and cash equivalents	$ 68,784	$ 115,516
Accounts receivable - related party	36,298	21,334
Prepaid expenses	6,981	8,133
Tax incentive receivable	7,857	7,689
Other current assets	1,561	1,667
Total Current Assets	121,481	154,339
Property, plant and equipment, net	112,580	109,266
Intangible assets, net	1,295	1,335
In-process research and development	752	742
Other assets	1,428	1,402
Equity method and other investments	6,326	6,326
Right-of-use assets - operating leases, net	19,427	20,109
Right-of-use assets - finance leases, net	24,851	24,718
TOTAL ASSETS	$ 288,140	$ 318,237
LIABILITIES AND SHAREHOLDERS' EQUITY		
CURRENT LIABILITIES:		
Accounts payable	$ 29,755	$ 16,616
Accrued expenses	31,067	39,818
Lease obligations, current	4,018	3,884
Deferred revenue - related party, current	13,693	15,123
Other current liabilities	2,571	6,631
Total Current Liabilities	81,104	82,072
Deferred revenue - related party	26,425	27,436
Lease obligations	16,453	17,331
Asset retirement obligations	2,238	2,179
Deferred income tax liability	189	186
Note payable, net	71,301	71,033
Other long-term liabilities	208	262
TOTAL LIABILITIES	197,918	200,499
COMMITMENTS AND CONTINGENCIES (Note 10)		
SHAREHOLDERS' EQUITY:		
Ordinary Shares, $0.00003881 par value, 1,288,327,750 authorized, 48,686,263 and 48,477,209 shares issued and outstanding at March 31, 2023 and December 31, 2022, respectively	2	2
Capital in excess of par value	587,094	581,893
Accumulated other comprehensive income	3,694	6,047
Accumulated deficit	(500,568)	(470,204)
Total Shareholders' Equity	90,222	117,738
TOTAL LIABILITIES AND SHAREHOLDERS' EQUITY	$ 288,140	$ 318,237