MeiraGTx Reports Third Quarter 2022 Financial and Operational Results and Receives $25 Million Investment from Johnson & Johnson Innovation - JJDC, Inc.

November 10, 2022

-- Updated Positive Topline Data from the Phase 1/2 Trial of Botaretigene Sparoparvovec (AAV-RPGR) for the Treatment of X-linked Retinitis Pigmentosa (XLRP)

-- Now Dosing Patients in Phase 1 Trial Evaluating AAV-GAD Gene Therapy for Parkinson's Disease

-- Received $25.0 Million Investment from Johnson & Johnson Innovation – JJDC, Inc. ("JJDC"), the Investment Arm of Johnson and Johnson

-- Company to Provide Clinical Program Update for the Phase 1 AQUAx Trial of AAV-hAQP1 for the Treatment of Grade 2/3 Radiation-Induced Xerostomia in December 2022

LONDON and NEW YORK, Nov. 10, 2022 (GLOBE NEWSWIRE) -- MeiraGTx Holdings plc (Nasdaq: MGTX), a vertically integrated, clinical-stage gene therapy company, today announced financial results for the third quarter ended September 30, 2022, and provided an update on recent progress.

"As we head into year-end, we continue to make progress in advancing our clinical and preclinical pipeline," said Alexandria Forbes, Ph.D., president and chief executive officer of MeiraGTx. "This includes presenting data from our Phase 1/2 clinical study in XLRP at AAO, further demonstrating botaretigene sparoparvovec's potential to improve vision in patients with retinitis pigmentosa. We are currently dosing patients in the Phase 3 LUMEOS trial and remain on track for a BLA in 2024. We are also pleased with the $25 million investment from JJDC which will help us to further our programs and continue development of our internal capabilities."

"In addition, we are now dosing patients with adeno-associated virus (AAV) encoding glutamic acid decarboxylase (AAV-GAD), an investigational gene therapy product candidate for Parkinson's disease," Dr. Forbes continued. "And in our xerostomia program, we look forward to presenting updated data from our Phase 1 AQUAx trial later this year."

"We are also very excited by the progress of our technology and manufacturing platforms," stated Dr. Forbes. "We published 15 abstracts at ESGCT in October this year, highlighting the robustness of our riboswitch gene regulation technology for gene and cell therapies, as well as the depth of our proprietary promoter and manufacturing and process development platforms."

Recent Highlights and Anticipated Milestones

Botaretigene Sparoparvovec for the Treatment of XLRP:

- In October 2022, MeiraGTx presented positive data from the MGT009 Phase 1/2 clinical trial of the investigational gene therapy botaretigene sparoparvovec (formerly referred to as AAV-RPGR) for the treatment of patients with XLRP with disease-causing variants in the *RPGR* gene at the American Academy of Ophthalmology (AAO) 2022 Annual Meeting.
- Treatment with botaretigene sparoparvovec was found to have an acceptable safety profile and was well-tolerated, with no dose-limiting events.[1]
- Adverse events (AE) profile was anticipated and manageable, with most AEs related to the surgical delivery procedure, transient and resolved without intervention.[1] A total of three serious adverse events (SAEs) were observed in the overall Phase 1/2 MGT009 clinical study; two SAEs, which were previously reported, were observed in the dose-escalation phase of the study (n=10; one retinal detachment and one panuveitis in the low dose cohort), and a single additional SAE of increased intraocular pressure was observed in the dose escalation phase and resolved with treatment.[1]
- Sustained or increased functional improvements were demonstrated at six months post-treatment in multiple endpoints across each of the three domains of vision -- retinal function, visual function, and functional vision -- in patients treated with botaretigene sparoparvovec when compared to the randomized untreated control arm of the study.[1]
- Further sensitivity analysis was conducted on study participants by applying the Phase 3 LUMEOS (NCT04671433) study eligibility criteria that corroborated the endpoints selected for the Phase 3 study.[1] Currently, the LUMEOS study of botaretigene sparoparvovec for the treatment of patients with XLRP with disease-causing variants in the RPGR gene is actively dosing patients.
- MeiraGTx and Janssen Pharmaceuticals, Inc. (Janssen), one of the Janssen Pharmaceutical Companies of Johnson & Johnson, are jointly developing botaretigene sparoparvovec as part of a broader collaboration to develop and commercialize gene therapies for the treatment of inherited retinal diseases.

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

- MeiraGTx plans to present data from all four cohorts (n=12) in the unilateral dose escalation Phase 1 AQUAx trial

(NCT04043104) as well as data from the bilateral cohorts (n=12) in the fourth quarter of 2022.
- The Company has met with regulatory agencies and is incorporating their feedback as it plans to initiate a randomized, double-blind, placebo-controlled Phase 2 study in the coming months with material manufactured in its cGMP facility in London, United Kingdom.

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

- The Company is now dosing patients in the AAV-GAD Phase 1 study under a new IND using material manufactured in its cGMP facility in London, United Kingdom.
- The objective of the AAV-GAD trial (NCT05603312) is to evaluate the safety and tolerability of AAV-mediated delivery of glutamic acid decarboxylase (GAD) gene transfer into the subthalamic nuclei (STN) of participants with Parkinson's disease.

Riboswitch Gene Control Platform:

- The Company presented new data from its gene control platforms at the European Society of Gene and Cell Therapy (ESGCT) Annual Congress in October 2022, including its gene regulation technology applied to cell therapy for the first time, in this case the regulation of CAR-Ts.
- The Company's next generation riboswitch-based gene regulation platform can be used to precisely control gene expression with an unprecedented dynamic range using novel, synthetic and orally delivered small molecules.

Gene Therapy Manufacturing:

- MeiraGTx's wholly-owned facilities have now produced GMP clinical trial material for 6 different indications, using multiple AAV serotypes, including administration into the eye, salivary gland and central nervous system.
- The Company believes that bringing all aspects of testing and vector production in-house reduces regulatory risk, ensures the highest quality of products, lowers costs, and helps avoid bottlenecks in clinical development.
- In addition to its 30,000-square-foot facility in London, MeiraGTx now has a 150,000-square-foot plant in Shannon, Ireland which contains three facilities: one built to be flexible and scalable for viral vector production, another to manufacture plasmid DNA – the critical starting material for producing gene therapy products – and third, a Quality Control (QC) hub performing advanced biochemical quality control testing appropriate for commercialization.

Investment by JJDC:

- On November 9, 2022, JJDC purchased $25 million of MeiraGTx's ordinary shares in a private placement at the closing price of $6.68 per share.

In addition to the $25.0 million investment from JJDC, the Company had cash and cash equivalents of approximately $114.7 million as of September 30, 2022, as well as approximately $23.7 million in receivables due from Janssen from the third quarter of 2022. The Company believes it will have sufficient capital to fund operating expenses and capital expenditure requirements into the fourth quarter of 2024.

Financial Results

License revenue was $4.8 million for the three months ended September 30, 2022, compared to $6.9 million for the three months ended September 30, 2021. This decrease represents decreased amortization of the $100.0 million upfront payment from Janssen as well as amortization of the $30.0 million milestone payment received in connection with the Janssen Collaboration Agreement.

General and administrative expenses were $10.8 million for the three months ended September 30, 2022, compared to $7.9 million for the three months ended September 30, 2021. The increase of $2.9 million is primarily due to an increase of $0.9 million in consulting fees, $0.7 million in rent and facilities costs due to additional allocations to research and development, $0.6 million in legal and accounting fees, $0.6 million in share-based compensation, $0.3 million in payroll and payroll-related costs and $0.1 million in depreciation. These increases were partially offset by a decrease of $0.3 million in insurance costs.

Research and development expenses for the three months ended September 30, 2022, were $16.9 million, compared to $21.6 million for the three months ended September 30, 2021. The decrease of $4.7 million is primarily due to a decrease of $0.6 million in license fees, $0.4 million in rent and facilities costs, $0.9 million reduction of the estimated research and development tax credit refund and an increase of $7.1 million in research funding provided under the Collaboration Agreement with Janssen. These decreases were partially offset by an increase of $1.3 million in payroll and payroll-related costs, $1.0 million in share-based compensation, $1.0 million in costs related to the manufacture of material for the Company's clinical trials and $1.0 million in costs related to the Company's pre-clinical research and clinical trials.

Foreign currency loss was $12.8 million for the three months ended September 30, 2022, compared to a loss of $3.4 million for the three months ended September 30, 2021. The increase in the loss of $9.4 million was primarily due to an unrealized loss on the quarterly valuation of the Company's intercompany payables and receivables due to the strengthening of the U.S. dollar against the pound sterling and euro during the three months ended September 30, 2022.

Net loss attributable to ordinary shareholders for the quarter ended September 30, 2022, was $37.3 million, or $0.83 basic and diluted net loss per ordinary share, compared to a net loss attributable to ordinary shareholders of $25.9 million, or $0.59 basic and diluted net loss per ordinary share for the quarter ended September 30, 2021.

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 2022 milestones regarding our pre-clinical and clinical data and reporting of such data and the timing of results of data, including in light of the COVID-19 pandemic, 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 September 30, 2022, 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.

[1] Michaelides, M et al. Ph1/2 AAV5-RPGR (Botaretigene Sparoparvovec) Gene Therapy Trial in RPGR-associated X-linked Retinitis Pigmentosa (XLRP). Abstract #30071754. Presented at the 2022 American Academy of Ophthalmology Annual Meeting.

Contacts

Investors:
MeiraGTx
Investors@meiragtx.com

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 September 30,		For the Nine-Month Period Ended September 30,	
	2022	2021	2022	2021
License revenue - related party	$ 4,816	$ 6,948	$ 21,208	$ 16,658
Operating expenses:				
General and administrative	10,762	7,887	32,548	28,214
Research and development	16,862	21,613	63,960	53,512
Total operating expenses	27,624	29,500	96,508	81,726
Loss from operations	(22,808)	(22,552)	(75,300)	(65,068)
Other non-operating income (expense):				

Foreign currency loss	(12,838)	(3,367)	(25,911)	(4,600)
Interest income	288	33	345	188
Interest expense	(1,892)	(59)	(2,051)	(169)
Fair value adjustment	(34)	—	615	—
Net loss	(37,284)	(25,945)	(102,302)	(69,649)
Other comprehensive income:				
Foreign currency translation gain	8,772	2,669	18,062	1,991
Comprehensive loss	$ (28,512)	$ (23,276)	$ (84,240)	$ (67,658)
Net loss	$ (37,284)	$ (25,945)	$ (102,302)	$ (69,649)
Basic and diluted net loss per ordinary share	$ (0.83)	$ (0.59)	$ (2.29)	$ (1.58)
Weighted-average number of ordinary shares outstanding	44,687,635	44,170,299	44,620,900	44,094,873

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

	September 30, 2022	December 31, 2021
ASSETS		
CURRENT ASSETS:		
Cash and cash equivalents	$ 114,706	$ 137,703
Accounts receivable - related party	23,701	22,384
Prepaid expenses	10,171	8,102
Tax incentive receivable	4,897	12,634
Other current assets	1,561	2,420
Total Current Assets	155,036	183,243
Property, plant and equipment, net	93,620	75,860
Intangible assets, net	1,293	1,791
In-process research and development	682	783
Other assets	1,322	1,404
Equity method and other investments	6,656	6,656
Right-of-use assets - operating leases, net	19,913	22,782
Right-of-use assets - finance leases, net	22,890	27,645
TOTAL ASSETS	$ 301,412	$ 320,164
LIABILITIES AND SHAREHOLDERS' EQUITY		
CURRENT LIABILITIES:		
Accounts payable	$ 21,693	$ 15,348
Accrued expenses	24,774	27,586
Lease obligations, current	3,659	3,374
Deferred revenue - related party, current	18,878	21,820
Other current liabilities	3,359	—
Total Current Liabilities	72,363	68,128
Deferred revenue - related party	15,486	43,046
Lease obligations	17,458	20,359
Asset retirement obligations	2,081	2,081
Deferred income tax liability	171	196
Note payable, net	70,845	—
Other long-term liabilities	338	953
TOTAL LIABILITIES	178,742	134,763
COMMITMENTS (Note 10)		
SHAREHOLDERS' EQUITY:		
Ordinary Shares, $0.00003881 par value, 1,288,327,750 authorized, 44,725,678 and 44,548,925 shares issued and outstanding at September 30, 2022 and December 31, 2021, respectively	2	2

Capital in excess of par value	550,168	528,659
Accumulated other comprehensive income (loss)	15,391	(2,671)
Accumulated deficit	(442,891)	(340,589)
Total Shareholders' Equity	122,670	185,401
TOTAL LIABILITIES AND SHAREHOLDERS' EQUITY	$ 301,412	$ 320,164