MeiraGTx Reports Second Quarter 2023 Financial and Operational Results

August 10, 2023

- *Phase 3 LUMEOS clinical trial of botaretigene sparoparvovec (bota-vec, formerly AAV-RPGR) for the treatment of X-linked retinitis pigmentosa (XLRP) enrollment target has now been surpassed*
- *Announced positive clinical data from the completed AQUAx Phase 1 study of AAV2-hAQP1 for the treatment of grade 2/3 radiation-induced xerostomia (RIX), showing bilaterally treated participants reaching normal levels of whole saliva flow rate by 2 months post-treatment and persisting through the final Month 12 assessment in the study*
- *Initiated Phase 2 randomized, double-blind, placebo-controlled study of AAV2-hAQP1 for the treatment of grade 2/3 RIX*
- *Wholly-owned gene therapy manufacturing facility in Shannon, Ireland received Commercial MIA Authorization for QC Testing and is the first manufacturing site for gene therapy to receive a commercial license in Ireland*
- *Presented data on proprietary RiboCAR platform demonstrating improved CAR-T anti-cancer efficacy and durability at the 2023 American Society of Gene and Cell Therapy (ASGCT) Spotlight on Immuno-Oncology*

LONDON and NEW YORK, Aug. 10, 2023 (GLOBE NEWSWIRE) -- MeiraGTx Holdings plc (Nasdaq: MGTX), a vertically integrated, clinical stage gene therapy company, today announced financial and operational results for the second quarter ended June 30, 2023, and provided a corporate update.

“Progress across our programs and infrastructure continues to be extremely impressive,” said Alexandria Forbes, Ph.D., president and chief executive officer of MeiraGTx. “This includes further advancement of our three lead clinical programs, our riboswitch gene regulation technology platform, and our manufacturing platform. This quarter, we announced positive data from the completed AQUAx Phase 1 study, demonstrating clinically meaningful improvements in long term xerostomia patients, and we are currently enrolling and treating patients in our Phase 2 trial. In the Phase 3 LUMEOS trial of bota-vec for the treatment of XLRP, we have now surpassed the enrollment target and expect a BLA submission next year. In addition, we presented unprecedented preclinical data from our novel gene regulation platform at the ASGCT Annual and ASGCT Spotlight on Immuno-oncology meetings. We are particularly excited by our groundbreaking RiboCAR technology. RiboCAR enables precise and reversible regulation of CAR expression in response to small molecule inducers, with the regulated CAR-T showing increased activity and durability compared to CAR-T with constitutively active CAR, as well as significant implications for safety. We are excited to continue this work with leaders in the field in moving RiboCAR towards the clinic, particularly in the treatment of solid tumors where we believe RiboCAR’s benefits have the potential to transform patient outcomes.”

Dr. Forbes continued, “We continue to build on our unique end-to-end manufacturing capabilities with a commercial QC testing license for our state-of-the-art manufacturing facility in Shannon, Ireland. This is the first commercial license for a gene therapy facility in Ireland. This authorization allows us to accelerate the development of our pipeline and serve as a reliable QC testing facility for advanced therapies on a global scale, and provides the opportunity to add additional revenue from potential partners.”

Recent Development Highlights and Anticipated Milestones

Bota-vec for the Treatment of XLRP:

- Enrollment target in the pivotal Phase 3 LUMEOS clinical trial of bota-vec has been surpassed, with BLA submission expected in 2024.
- 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.

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

- Initiated a Phase 2 randomized, double-blind, placebo-controlled study with participants currently being enrolled and dosed.
- Reported clinically meaningful improvements from the AQUAx Phase 1 study in xerostomia symptoms measured by two different xerostomia patient reported outcome questionnaires (PROs) demonstrated across both unilaterally and bilaterally treated cohorts at 12 months:
    - Increases in whole saliva flow rates observed post-treatment, providing objective evidence of biological activity, reaching the normal range in bilaterally treated participants by 2 months and persisting through the Month 12 assessment.
    - Across assessments, greater improvements were observed in bilaterally treated participants compared to those treated unilaterally.
    - Early long-term follow-up data suggest durability of improvement to at least 3 years post-treatment.

    - AAV2-hAQP1 appears safe and well tolerated at each dose tested.
    - The strong safety and encouraging, clinically meaningful activity data support further clinical development of AAV2-hAQP1.
- The Company anticipates presenting the full data from the AQUAx Phase 1 study at the next appropriate medical meeting.

Wholly-Owned Gene Therapy Manufacturing Facility in Shannon, Ireland has Received Commercial MIA Authorization for QC Testing

- The QC facility in Shannon, Ireland performs advanced biochemical quality control testing for release and stability testing for MeiraGTx's and its partner's programs.
- Unique in its scale and integrated capabilities and stretching over 150,000 square feet, the GMP Shannon facility is Ireland's first commercial-scale gene therapy manufacturing site and contains facilities for flexible and scalable viral vector production for clinical and commercial supply as well as a facility for plasmid DNA production in addition to the GMP licensed QC facility.

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

- The Company is 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:

- Presented data at the 2023 ASGCT Spotlight on Immuno-Oncology
    - RiboCAR-T cell activity can be precisely tuned and "remotely" controlled to improve the efficacy, durability, and safety of CAR-T cell therapy.
    - Proprietary switch allows the Company to precisely and reversibly control the expression of CAR in a dose-response to novel small molecule inducers to unprecedented levels, from undetectable at baseline to at least as high as levels of constitutively expressed CAR driven by the small molecule dose.
- Presentations at the ASGCT 2023 Annual Meeting
    - The Company exhibited nine poster presentations highlighting the depth and novelty of MeiraGTx's technology platforms for gene and cell therapy.

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

As of June 30, 2023, MeiraGTx had cash and cash equivalents of approximately $92.8 million, as well as approximately $32.7 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 $92.8 million as of June 30, 2023, compared to $115.5 million as of December 31, 2022.

License revenue was $3.5 million for the quarter ended June 30, 2023, compared to $10.8 million for the quarter ended June 30, 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 $12.4 million for the three months ended June 30, 2023, compared to $10.5 million for the three months ended June 30, 2022. The increase of $1.9 million was primarily due to an increase in legal and accounting fees, payroll and payroll-related costs, share-based compensation and other office related costs. These increases were partially offset by decreases in insurance costs and consulting fees.

Research and development expenses for the three months ended June 30, 2023, were $19.9 million, compared to $24.0 million for the three months ended June 30, 2022. The decrease of $4.1 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, manufacturing costs related to lower production volumes, other research and development expenses primarily due to a decrease in share-based compensation, and 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 our Phase 2 clinical trial for AAV-hAQP1.

Foreign currency gain was $1.9 million for the three months ended June 30, 2023, compared to a loss of $10.4 million for the three months ended June 30, 2022. The change of $12.3 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 June 30, 2023.

Net loss attributable to ordinary shareholders for the quarter ended June 30, 2023 was $29.6 million, or $0.53 basic and diluted net loss per ordinary share, compared to a net loss attributable to ordinary shareholders of $34.0 million, or $0.76 basic and diluted net loss per ordinary share for the

quarter ended June 30, 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 June 30, 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.

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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)

	Forthe Three-Month Period Ended June 30,		Forthe Six-Month Period Ended June 30,	
	2023	2022	2023	2022
License revenue - related party	$ 3,540	$ 10,759	$ 6,874	$ 16,392
Operating expenses:				
General and administrative	12,388	10,518	25,160	21,786
Research and development	19,937	23,999	42,259	47,098
Total operating expenses	32,325	34,517	67,419	68,884

Loss from operations	(28,785)	(23,758)	(60,545)	(52,492)
Other non-operating income (expense):				
Foreign currency gain (loss)	1,905	(10,426)	5,762	(13,073)
Interest income	655	41	1,200	57
Interest expense	(3,355)	(82)	(6,415)	(159)
Fair value adjustment	(1)	252	53	649
Net loss	(29,581)	(33,973)	(59,945)	(65,018)
Other comprehensive (loss) income:				
Foreign currency translation (loss) gain	(2,541)	7,357	(4,894)	9,290
Comprehensive loss	$ (32,122)	$ (26,616)	$ (64,839)	$ (55,728)
Net loss	$ (29,581)	$ (33,973)	$ (59,945)	$ (65,018)
Basic and diluted net loss per ordinary share	$ (0.53)	$ (0.76)	$ (1.15)	$ (1.46)
Weighted-average number of ordinary shares outstanding	55,349,534	44,668,240	52,012,382	44,585,239

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

	June30, 2023	December31, 2022
ASSETS		
CURRENT ASSETS:		
Cash and cash equivalents	$ 92,773	$ 115,516
Accounts receivable - related party	32,690	21,334
Prepaid expenses	8,108	8,133
Tax incentive receivable	8,026	7,689
Other current assets	1,547	1,667
Total Current Assets	143,144	154,339
Property, plant and equipment, net	114,004	109,266
Intangible assets, net	1,252	1,335
In-process research and development	753	742
Other assets	1,465	1,402
Equity method and other investments	6,326	6,326
Right-of-use assets - operating leases, net	18,693	20,109
Right-of-use assets - finance leases, net	24,626	24,718
TOTAL ASSETS	$ 310,263	$ 318,237
LIABILITIES AND SHAREHOLDERS' EQUITY		
CURRENT LIABILITIES:		
Accounts payable	$ 28,497	$ 16,616
Accrued expenses	24,889	39,818
Lease obligations, current	4,126	3,884
Deferred revenue - related party, current	12,058	15,123
Other current liabilities	2,233	6,631
Total Current Liabilities	71,803	82,072
Deferred revenue - related party	25,364	27,436
Lease obligations	15,508	17,331
Asset retirement obligations	2,301	2,179
Deferred income tax liability	189	186
Note payable, net	71,571	71,033
Other long-term liabilities	—	262
TOTAL LIABILITIES	186,736	200,499

COMMITMENTS AND CONTINGENCIES (Note 10)		
SHAREHOLDERS' EQUITY:		
Ordinary Shares, $0.00003881 par value, 1,288,327,750 authorized, 59,535,314 and 48,477,209 shares issued and outstanding at June 30, 2023 and December 31, 2022, respectively	2	2
Capital in excess of par value	652,521	581,893
Accumulated other comprehensive income	1,153	6,047
Accumulated deficit	(530,149)	(470,204)
Total Shareholders' Equity	123,527	117,738
TOTAL LIABILITIES AND SHAREHOLDERS' EQUITY	$ 310,263	$ 318,237