MeiraGTx Reports Second Quarter 2022 Financial and Operational Results

August 11, 2022

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

-- Financing Secured by Manufacturing Facilities Extends Cash Runway to Fourth Quarter 2024

LONDON and NEW YORK, Aug. 11, 2022 (GLOBE NEWSWIRE) -- MeiraGTx Holdings plc (Nasdaq: MGTX), a vertically integrated, clinical-stage gene therapy company, today announced financial results for the second quarter ended June 30, 2022, and provided an update on recent progress.

“Over the past quarter we have continued to advance our gene therapy programs and platforms, including the recently announced positive topline data from the Phase 1/2 clinical study demonstrating that our investigational gene therapy, botaretigene sparoparvovec, has the potential to improve vision in patients with retinitis pigmentosa,” said Alexandria Forbes, Ph.D., president, and chief executive officer of MeiraGTx. “We look forward to presenting additional data from this study at the American Academy of Ophthalmology (AAO) annual meeting this year as we continue to enroll the pivotal Phase 3 Lumeos clinical trial of botaretigene sparoparvovec with our partner Janssen.”

Dr. Forbes continued, “We also recently announced a financing of up to $100 million with one of our largest shareholders, extending our cash runway to the fourth quarter of 2024. Our wholly-owned manufacturing facilities collateralized this transaction, enabling us to access minimally dilutive capital while retaining the significant and growing value of our proprietary programs and transformative genetic medicine platforms.”

Recent Highlights and Anticipated Milestones

Botaretigene Sparoparvovec for the Treatment of XLRP:

- In June 2022, MeiraGTx announced positive topline data from the MGT009 Phase 1/2 clinical trial of botaretigene sparoparvovec (formerly referred to as AAV-RPGR) for the treatment of patients with XLRP with disease-causing variants in the *RPGR* gene.
- Treatment with botaretigene sparoparvovec was found to be generally safe and well-tolerated, with no dose-limiting events.
- Significant improvements were demonstrated 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 at 6 months post-treatment.
- Full MGT009 data will be presented at the AAO annual meeting, being held September 30 – October 3, 2022, in Chicago, IL.
- The Company is currently enrolling patients in the Phase 3 Lumeos clinical trial of botaretigene sparoparvovec and targeting a Biologics License Application (BLA) filing in 2024.
- 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-CNGB3 and AAV-CNGA3 for the Treatment of Achromatopsia (ACHM):

- Development partner Janssen plans to initiate a Phase 2b clinical trial for the treatment of ACHM associated with mutations in the *CNGB3* gene in 2022 followed by a Phase 2b clinical trial for the treatment of ACHM associated with mutations in the *CNGA3* gene in early 2023.
- Both ACHM clinical trials to be initiated by Janssen will use material manufactured at MeiraGTx’s cGMP facility in London, United Kingdom.

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 as well as data from the bilateral cohorts (n=12) in the fourth quarter of 2022.
- The Company plans to initiate a randomized, double-blind, placebo-controlled Phase 2 study by the end of 2022 with material manufactured in its cGMP facility in London, United Kingdom.

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

- The Company expects to initiate enrollment in its study of AAV-GAD during the second half of 2022 with material manufactured in its cGMP facility in London, United Kingdom.

Gene Control Platforms:

- The Company will present new data from its gene regulation platforms at medical meetings in the second half of 2022.
- MeiraGTx is advancing several small molecule candidates from its gene regulation platform with the aim of initiating first-in-human safety and tolerability studies this year.
- Novel regulation platform can be used to precisely control gene expression in cell therapy, gene editing, with any gene and any vector with unprecedented dynamic range using an oral small molecule.

Financing Agreement with Perceptive for Up to $100 Million:

- In August 2022, MeiraGTx and Perceptive announced a senior secured financing arrangement for up to $100 million secured by MeiraGTx's wholly-owned manufacturing facilities in London, United Kingdom and Shannon, Ireland.
- The Company received $75 million upon closing and may request an additional $25 million during the first two years of the term under the same terms and collateral, subject to the lender's approval.

In addition to the $75.0 million gross proceeds from this recent financing, the Company had cash and cash equivalents of approximately $72.1 million as of June 30, 2022, as well as approximately $15.9 million in receivables due from Janssen from the second 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 $10.8 million for the three months ended June 30, 2022, compared to $5.1 million for the three months ended June 30, 2021. This increase represents increased amortization of the $100.0 million upfront payment as well as amortization of the $30.0 million milestone payment received in connection with the Collaboration Agreement.

General and administrative expenses were $10.5 million for the three months ended June 30, 2022, compared to $10.4 million for the three months ended June 30, 2021. The increase of $0.1 million was primarily due to an increase of $1.0 million in share-based compensation, $0.4 million in legal and accounting fees, $0.1 million in depreciation and $0.2 million in other office related costs. These increases were partially offset by decreases of rent and facilities costs of $0.9 million due to additional allocations to research and development, $0.4 million in insurance costs and $0.3 million in payroll and payroll-related costs.

Research and development expenses for the three months ended June 30, 2022 were $24.0 million, compared to $15.2 million for the three months ended June 30, 2021. The increase of $8.8 million was primarily due to an increase of $2.2 million in payroll and payroll-related costs, $1.9 million in costs related to the manufacture of material for the Company's clinical trials, $1.1 million in share-based compensation, $1.0 million in rent and facilities costs, $0.4 million in costs related to the Company's pre-clinical research and clinical trials, $0.2 million in other research and development costs and a decrease of $2.3 million in research funding provided under the Collaboration Agreement with Janssen. These increases were partially offset by a decrease of $0.2 million in license fees and $0.1 million in depreciation.

Foreign currency loss was $10.4 million for the three months ended June 30, 2022 compared to a gain of $0.4 million for the three months ended June 30, 2021. The change in the amount of $10.8 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 June 30, 2022.

Net loss attributable to ordinary shareholders for the quarter ended June 30, 2022 was $34.0 million, or $0.76 basic and diluted net loss per ordinary share, compared to a net loss attributable to ordinary shareholders of $20.1 million, or $0.46 basic and diluted net loss per ordinary share for the quarter ended June 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 which 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 June 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.

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 June 30,		For the Six-Month Period Ended June 30,	
	2022	2021	2022	2021
License revenue - related party	$ 10,759	$ 5,116	$ 16,392	$ 9,711
Operating expenses:				
General and administrative	10,518	10,409	21,786	20,327
Research and development	23,999	15,190	47,098	31,900
Total operating expenses	34,517	25,599	68,884	52,227
Loss from operations	(23,758)	(20,483)	(52,492)	(42,516)
Other non-operating income (expense):				
Foreign currency (loss) gain	(10,426)	381	(13,073)	(1,234)
Interest income	41	67	57	156
Interest expense	(82)	(51)	(159)	(110)
Fair value adjustment	252	—	649	—
Net loss	(33,973)	(20,086)	(65,018)	(43,704)
Other comprehensive income (loss):				
Foreign currency translation gain (loss)	7,357	(407)	9,290	(678)
Total comprehensive loss	$ (26,616)	$ (20,493)	$ (55,728)	$ (44,382)
Net loss	$ (33,973)	$ (20,086)	$ (65,018)	$ (43,704)
Basic and diluted net loss per ordinary share	$ (0.76)	$ (0.46)	$ (1.46)	$ (0.99)
Weighted-average number of ordinary shares outstanding	44,668,240	44,137,773	44,585,239	44,056,535

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

	June 30, 2022	December 31, 2021
ASSETS		
CURRENT ASSETS:		

Cash and cash equivalents	$	72,108	$	137,703
Accounts receivable - related party		15,942		22,384
Prepaid expenses		6,682		8,102
Tax incentive receivable		6,350		12,634
Other current assets		3,330		2,420
Total Current Assets		104,412		183,243
Property, plant and equipment, net		91,388		75,860
Intangible assets, net		1,474		1,791
In-process research and development		723		783
Other assets		1,505		1,404
Equity method and other investments		6,656		6,656
Right-of-use assets - operating leases, net		21,538		22,782
Right-of-use assets - finance leases, net		24,765		27,645
TOTAL ASSETS	$	252,461	$	320,164
LIABILITIES AND SHAREHOLDERS' EQUITY				
CURRENT LIABILITIES:				
Accounts payable	$	18,111	$	15,348
Accrued expenses		23,572		27,586
Lease obligations, current		3,634		3,374
Deferred revenue - related party, current		21,205		21,820
Other current liabilities		983		—
Total Current Liabilities		67,505		68,128
Deferred revenue - related party		21,337		43,046
Lease obligations		19,063		20,359
Asset retirement obligations		2,099		2,081
Deferred income tax liability		181		196
Other long-term liabilities		304		953
TOTAL LIABILITIES		110,489		134,763
COMMITMENTS (Note 9)				
SHAREHOLDERS' EQUITY:				
Ordinary Shares, $0.00003881 par value, 1,288,327,750 authorized, 44,710,678 and 44,548,925 shares issued and outstanding at June 30, 2022 and December 31, 2021, respectively		2		2
Capital in excess of par value		540,958		528,659
Accumulated other comprehensive income (loss)		6,619		(2,671)
Accumulated deficit		(405,607)		(340,589)
Total Shareholders' Equity		141,972		185,401
TOTAL LIABILITIES AND SHAREHOLDERS' EQUITY	$	252,461	$	320,164