MeiraGTx Reports Fourth Quarter and Full Year 2020 Financial and Operational Results

March 11, 2021

- Preparing to Initiate Phase 3 Lumeos Clinical Trial in AAV-RPGR
- Announced Positive Preliminary Clinical Data from AQUAx AAV-hAQP1 Phase 1 Trial for Treatment of Grade 2/3 Xerostomia
- Completed Build of Internal cGMP Plasmid and DNA Manufacturing Facility
- Strengthened Balance Sheet and Extended Cash Runway into Mid-2023
- First in-vivo Data from Riboswitch Gene Regulation Platform to be Presented in 2021

LONDON and NEW YORK, March 11, 2021 (GLOBE NEWSWIRE) -- MeiraGTx Holdings plc (Nasdaq: MGTX), a vertically integrated, clinical stage gene therapy company, today announced financial and operational results for the fourth quarter and full year ended December 31, 2020 and provided an update on recent progress.

"MeiraGTx experienced another year of significant progress in 2020 as we advanced our clinical programs, presented positive clinical data from our X-linked retinitis pigmentosa and xerostomia clinical programs and further expanded our industry-leading gene therapy vectorology and manufacturing capabilities," said Alexandria Forbes, Ph.D., president and chief executive officer of MeiraGTx. "With the initiation of our first pivotal trial approaching for AAV-RPGR, our recent announcement of Fast Track designation for AAV-CNGA3 for the treatment of achromatopsia, and positive data from the first cohort in our AQUAx phase 1 clinical trial for the treatment of grade 2/3 xerostomia, we entered 2021 with significant momentum."

Dr. Forbes continued, "Our state-of-the-art facilities and manufacturing infrastructure allow us to execute our proprietary, commercial AAV production process, enabling us to accelerate clinical development and the delivery of innovative therapies to patients. In addition, this year we look forward to sharing *in-vivo* data from our riboswitch gene regulation platform which we believe to be an unprecedented and transformational technology."

As of December 31, 2020, MeiraGTx had cash and cash equivalents of approximately $209 million, as well as approximately $38 million due in receivables in the next 30 days from development partner Janssen Pharmaceuticals, Inc. (Janssen), one of the Janssen Pharmaceutical Companies of Johnson & Johnson, as part of a broader collaboration to develop and commercialize gene therapies for the treatment of inherited retinal diseases. The Company believes it will have sufficient capital to fund operating expenses and capital expenditure requirements into mid-2023.

Recent Clinical Development Highlights and Anticipated 2021 Milestones

AAV-AQP1 for the Treatment of Grade 2/3 Radiation-Induced Xerostomia:

- MeiraGTx reported preliminary data from the Phase 1 AQUAx clinical trial in December 2020.
  - Of the three patients treated in Cohort 1, one patient reached the 12-month assessment and two passed the six-month assessment. In all patients, the investigational gene therapy AAV-hAQP1 has been well tolerated with no dose limiting toxicity and no serious adverse events reported.
  - Encouraging responses have been seen in patient-reported measures of xerostomia symptoms and in salivary output in the patients treated in Cohort 1.
  - Complete resolution of symptoms was observed in the patient who has reached the 12-month timepoint.
- MeiraGTx continues to activate clinical trial sites in the Company's Phase 1 AQUAx study, with two sites re-opened after shutdowns due to COVID-19, and all five sites are anticipated to be open and enrolling patients in the first half of 2021.
- The single center Phase 1 dose-finding study of AAV-AQP1 at the National Institutes of Health (NIH) also continues to enroll patients. Enrollment in the fourth dose escalation cohort is now ongoing.

AAV-RPGR for the Treatment of X-Linked Retinitis Pigmentosa (XLRP):

- MeiraGTx and Janssen are preparing to initiate the Phase 3 Lumeos clinical trial.
- In 2020, MeiraGTx and Janssen were granted Priority Medicines (PRIME) and Advanced Therapy Medicinal Product (ATMP) designations for AAV-RPGR.
- In 2020, MeiraGTx and Janssen announced positive 6-, 9- and 12-month data from the Phase 1/2 clinical study (MGT009) of AAV-RPGR at the American Society of Retina Specialists (ASRS) Annual Meeting, the European Society of Retina Specialists (EURETINA), and the American Academy of Ophthalmology Annual Meeting:
  - Data from each time point demonstrated that patients treated with low and intermediate dose AAV-RPGR experienced statistically significant improvement in retinal sensitivity. Nine-month data also indicated significant improvement in vision-guided mobility. At 12-months, six of seven patients continued to show improved or stable vision in the treated eye.

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

- MeiraGTx anticipates filing an Investigational New Drug application (IND) by the third quarter of 2021, with material that

has been manufactured with MeiraGTx's in-house proprietary manufacturing process at the MeiraGTx cGMP manufacturing facility in London.

AAV-RPE65 for the Treatment of *RPE65*-associated Retinal Dystrophy:

- MeiraGTx anticipates initiating a Phase 3 pivotal trial of AAV-RPE65 in the second half of 2021.

AAV-CNGB3 and AAV-CNGA3 for the Treatment of Achromatopsia (ACHM):

- MeiraGTx and Janssen continue to advance the Company's ongoing clinical development of AAV-CNGB3 and AAV-CNGA3 for the treatment of ACHM associated with mutations in the *CNGB3* and *CNGA3* genes.
  - On January 26, 2021 the U.S. Food and Drug Administration (FDA) granted Fast Track designation to the Company's AAV-CNGA3 gene therapy product candidate for the treatment of ACHM caused by mutations in the *CNGA3* gene.
  - MeiraGTx and Janssen have now completed dosing of both adults and pediatric patients in the Phase 1/2 dose escalation study of AAV-CNGA3 and expect to provide an update on further clinical studies for both AAV-CNGB3 and AAV-CNGA3 later in 2021.

Riboswitch Gene Regulation Platform:

- MeiraGTx expects to present *in-vivo* data from its proprietary riboswitch gene regulation platform in the second half of 2021, demonstrating regulation of multiple therapeutic genes in multiple tissues.

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

Recent Corporate Development Highlights

Second Viral Vector Manufacturing Facility and Plasmid and DNA Production Facility:

- The Company expanded its industry-leading manufacturing capabilities by acquiring and building a second wholly owned cGMP viral vector manufacturing facility as well as a cGMP plasmid and DNA production facility located in Shannon, Ireland.
- The campus encompasses approximately 150,000 sq. ft. serving numerous functions: high capacity cGMP manufacturing hub, clinical supply storage, QC laboratories for global release, up to ten flexible and scalable viral vector suites, fully scalable automated fill and finish, an extensive warehouse and a separate internal cGMP plasmid and DNA manufacturing facility.
- Construction of the cGMP plasmid and DNA manufacturing facility has been completed, with the cGMP viral vector manufacturing facility expected to be completed by the end of 2021.

Expanding Clinical, Regulatory, Manufacturing, MSAT and Preclinical Development Teams:

- The Company continues to increase the number of personnel across key functional areas to support its broad pipeline of optimized investigational gene therapies. The MeiraGTx team now includes more than 215 full-time employees.

Financial Results

Cash, cash equivalents and restricted cash were $209.5 million as of December 31, 2020, compared to $227.4 million as of December 31, 2019.

License revenue was $15.6 million for the year ended December 31, 2020, compared to $13.3 million for the year ended December 31, 2019. This increase represents the increased amortization of the $100.0 million upfront payment received in connection with the Janssen collaboration agreement.

General and administrative expenses were $44.2 million for the year ended December 31, 2020, compared to $46.7 million for the year ended December 31, 2019. The decrease of $2.5 million was primarily due to decreases in payroll and payroll related costs and travel expenses which was partially offset by increases in insurance, share-based compensation, rent, professional fees and other general and administrative expenses.

Research and development expenses for the years ended December 31, 2020 and 2019 were as follows (in millions):

	2020	2019	Change
Gross research and development expenses	$ 96.6	$ 65.0	$ 31.6
Janssen reimbursements	(57.4)	(28.1)	(29.3)
Tax incentive reimbursement	(5.3)	(12.0)	6.7
Research and development expenses	$ 33.9	$ 24.9	$ 9.0

Gross research and development expenses for the year ended December 31, 2020 increased $31.6 million as compared to the prior year primarily due to an increase in manufacturing of our clinical trial materials, payroll and payroll related costs, acquired research and development, depreciation, rent and facility costs and share-based compensation, which was partially offset by a decrease in research and clinical trial costs related to our ophthalmology and salivary gland programs.

Reimbursements under the Janssen collaboration agreement for the year ended December 31, 2020 increased $29.3 million as compared to the prior year primarily due to an increase in activity in the programs licensed under the Janssen collaboration agreement.

Tax incentive reimbursement for the year ended December 31, 2020 decreased $6.7 million as compared to the prior year primarily due to the 2018 and 2019 U.K. refundable research and development credit being recorded in 2019. In 2020, only the 2020 U.K. refundable research and development credit was recorded.

Foreign currency gain was $3.4 million for the year ended December 31, 2020 compared to a gain of $3.2 million for the year ended December 31, 2019. The change of $0.2 million was primarily due to a strengthening of the pound sterling and euro against the U.S. dollar in 2020.

Interest income was $1.3 million for the year ended December 31, 2020 compared to $0.4 million for the year ended December 31, 2019. The increase was due to a higher average cash balance during 2020 and a reallocation of funds into an account earning a higher interest rate.

Net loss attributable to ordinary shareholders for the year ended December 31, 2020 was $58.0 million, or $1.54 basic and diluted net loss per ordinary share, compared to a net loss attributable to ordinary shareholders of $54.8 million, or $1.65 basic and diluted net loss per ordinary share for the year ended December 31, 2019.

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, as well as a potentially transformative gene regulation technology. 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, including inherited retinal diseases and large degenerative diseases, neurodegenerative diseases and severe forms of xerostomia. Though initially focusing on the eye, central nervous system and salivary gland, MeiraGTx intends to expand its focus in the future 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 2021 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," "intend," "plan," "believe," "project," "forecast," "estimate," "may," "should," "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, 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 Annual Report on Form 10-K for the year ended December 31, 2020, 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
CONSOLIDATED STATEMENTS OF OPERATIONS AND COMPREHENSIVE LOSS

	For the Year Ended December 31,	
	2020	2019
License revenue - related party	$ 15,562,985	$ 13,291,956
Operating expenses:		
General and administrative	44,206,921	46,684,297
Research and development	33,910,481	24,875,659
Total operating expenses	78,117,402	71,559,956
Loss from operations	(62,554,417)	(58,268,000)
Other non-operating income (expense):		
Foreign currency gain	3,426,152	3,199,774
Interest income	1,275,464	370,603
Interest expense	(139,203)	(48,612)
Net loss	(57,992,004)	(54,746,235)
Other comprehensive loss:		
Foreign currency translation	(3,102,864)	(2,087,708)
Total comprehensive loss	$ (61,094,868)	$ (56,833,943)
Net loss	$ (57,992,004)	$ (54,746,235)
Basic and diluted net loss per ordinary share	$ (1.54)	$ (1.65)
Weighted-average number of ordinary shares outstanding	37,724,189	33,161,860

MEIRAGTX HOLDINGS PLC AND SUBSIDIARIES
CONSOLIDATED BALANCE SHEETS

	December 31, 2020	December 31, 2019
ASSETS		
CURRENT ASSETS:		
Cash and cash equivalents	$ 209,520,355	$ 227,233,384
Accounts receivable - related party	38,479,371	23,337,377
Prepaid expenses	7,081,747	4,464,085
Tax incentive receivable	12,930,062	11,974,437
Other current assets	4,564,441	1,970,585
Total Current Assets	272,575,976	268,979,868
Property and equipment, net	44,041,903	23,858,108
Intangible assets, net	2,119,011	—
In-process research and development	852,085	777,655
Security deposits	812,344	951,138
Restricted cash	—	123,376
Other assets	213,722	195,053
Right-of-use assets	43,082,359	29,002,448
TOTAL ASSETS	$ 363,697,400	$ 323,887,646
LIABILITIES AND SHAREHOLDERS' EQUITY		
CURRENT LIABILITIES:		
Accounts payable	$ 7,134,204	$ 3,759,339
Accrued expenses	20,860,820	18,083,757
Lease obligations, current	2,582,999	1,674,210
Deferred revenue - related party, current	23,544,583	25,678,515
Other current liabilities	24,453	—
Total Current Liabilities	54,147,059	49,195,821
Deferred revenue - related party	49,297,194	60,535,576
Lease obligations	19,665,841	21,504,340
Asset retirement obligations	1,814,338	1,654,755

Deferred income tax liability	213,722	195,053
TOTAL LIABILITIES	125,138,154	133,085,545
COMMITMENTS		
SHAREHOLDERS' EQUITY:		
Ordinary Shares, $0.00003881 par value, 1,288,327,750 authorized, 44,189,150 and 36,791,906 shares issued and outstanding at December 31, 2020 and 2019, respectively	1,716	1,429
Capital in excess of par value	504,482,392	395,630,666
Accumulated other comprehensive loss	(4,896,906)	(1,794,042)
Accumulated deficit	(261,027,956)	(203,035,952)
Total Shareholders' Equity	238,559,246	190,802,101
TOTAL LIABILITIES AND SHAREHOLDERS' EQUITY	$ 363,697,400	$ 323,887,646