MeiraGTx Reports Fourth Quarter and Full Year 2021 Financial and Operational Results

March 10, 2022

- Phase 3 Lumeos Clinical Trial of botaretigene sparoparvovec (AAV-RPGR) for the Treatment of X-linked Retinitis Pigmentosa (XLRP) Enrolling and Dosing Patients
- Received $30 Million Cash Milestone Payment from Janssen Pharmaceuticals, Inc.
- Positive Preliminary Clinical Data from AQUAx AAV-hAQP1 Phase 1 Trial for Treatment of Grade 2/3 Radiation-Induced Xerostomia Announced
- First In Vivo Data from Riboswitch Gene Regulation Platform at R&D Day Presented

LONDON and NEW YORK, March 10, 2022 (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, 2021, and provided a corporate update.

“MeiraGTx had an exceptional year in 2021, marked by advancements in all aspects of our pipeline and technology platforms,” said Alexandria Forbes, Ph.D., president and chief executive officer of MeiraGTx. “We initiated our first pivotal study with our partner Janssen for the treatment of XLRP using drug product manufactured in-house at our cGMP facility using our proprietary manufacturing process, we announced positive preliminary clinical data from our wholly-owned grade 2/3 xerostomia program and we presented the first *in vivo* data from our transformative gene regulation platform.”

“This progress is enabled by the broad end-to-end capabilities we have focused on building at the Company since inception,” continued Dr. Forbes. “We have established leading capabilities in vector engineering and optimization, viral vector production process development, manufacturing and quality, all while developing our synthetic biology platform which for the first time allows precise and specific control of genetic medicines using novel orally dosed small molecules.”

Recent Development Highlights and Anticipated 2022 Milestones

Botaretigene Sparoparvovec (AAV-RPGR) for the Treatment of XLRP:

- MeiraGTx, in collaboration with Janssen Pharmaceuticals, Inc. (Janssen), one of the Janssen Pharmaceutical Companies of Johnson & Johnson, is dosing and enrolling patients in the Phase 3 Lumeos clinical trial of botaretigene sparoparvovec.
- MeiraGTx received a $30 million payment from Janssen for a clinical milestone in the Phase 3 Lumeos trial of botaretigene sparoparvovec.

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

- MeiraGTx reported positive preliminary data from the Phase 1 AQUAx clinical trial in December 2021.
  - Clinically meaningful improvements in xerostomia symptoms and disease burden in two validated Patient-Reported Outcome (PRO) measures were demonstrated.
  - 6 of the 7 participants through 90-day assessments following treatment achieved clinically meaningful improvement in symptoms using both the McMaster Global Rate of Change PRO and the Xerostomia Questionnaire.
  - One participant with the maximum response evaluable at 12 months has now reached 24 months and the same level of response/xerostomia symptom improvement was maintained.
  - AAV-hAQP1 appears safe and well-tolerated at each dose tested.
- MeiraGTx completed treatment of patients in all four cohorts of the unilateral dose escalation (n=12) Phase 1 AQUAx trial in the fourth quarter of 2021.
- Four cohorts of dose escalating bilateral treatment (n=12) were added to the protocol to further assess potential efficacy. Treatment of all bilateral patients was completed in the first quarter of 2022.
- Based on the safety and efficacy profile of AAV-hAQP1 in the AQUAx Phase 1 study and regulatory precedent, the Company intends to initiate a randomized, double-blind, placebo-controlled Phase 2 study evaluating two active doses of AAV-hAQP1 by the end of 2022.

Riboswitch Gene Regulation Platform:

- MeiraGTx presented data from its proprietary riboswitch gene regulation platform in December 2021 demonstrating regulation of multiple therapeutic genes in multiple tissues *in vitro* and *in vivo*, as well as *in vivo* models showing dose responsive physiological effects indicative of potential therapeutic activity.
- Transformative riboswitch technology platform has unprecedented dynamic range of greater than 5,000-fold.
- Proprietary technology platform allows precise and specific control of gene therapy expression levels via dose-response to orally delivered small molecules.

- MeiraGTx has developed a library of novel small molecules that tightly regulate aptamer driven cassettes with drug properties designed specifically for the regulation of different genes in different tissues.
- This technology is applicable to the control of any gene in the context of any vector, including both gene editing and RNA editing.

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

- MeiraGTx anticipates filing an Investigational New Drug application (IND) in the coming weeks, with material that has been manufactured with our proprietary manufacturing process at the MeiraGTx cGMP manufacturing facility in London.

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

- With partner Janssen, MeiraGTx expects to initiate later stage clinical studies in 2022 for both AAV-CNGB3 and AAV-CNGA3 for the treatment of ACHM associated with mutations in the *CNGB3* and *CNGA3* genes.

Manufacturing and Process Development:

- MeiraGTx successfully manufactured cGMP material for six different clinical programs, including three for Janssen-partnered programs, highlighting the breadth and expertise of the Company's manufacturing and quality infrastructure.

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

As of December 31, 2021, MeiraGTx had cash and cash equivalents of approximately $137.7 million, as well as approximately $22.4 million due in receivables in the next 30 days from development partner Janssen 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 through the second quarter of 2023.

Financial Results

Cash and cash equivalents were $137.7 million as of December 31, 2021, compared to $209.5 million as of December 31, 2020.

License revenue was $37.7 million for the year ended December 31, 2021, compared to $15.6 million for the year ended December 31, 2020. This increase represents the 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 Janssen collaboration agreement.

General and administrative expenses were $43.8 million for the year ended December 31, 2021, compared to $44.2 million for the year ended December 31, 2020. The decrease of $0.4 million was primarily due to a decrease in share-based compensation and payroll and payroll related costs, which was partially offset by increases in insurance, facility costs, professional fees and other general and administrative expenses.

Research and development expenses for the years ended December 31, 2021 and 2020 were as follows (in millions):

	2021	2020	Change
Gross research and development expenses	$ 141.5	$ 96.6	$ 44.9
Janssen reimbursements	(69.0)	(57.4)	(11.6)
Tax incentive reimbursement	(5.4)	(5.3)	(0.1)
Research and development expenses	$ 67.1	$ 33.9	$ 33.2

Gross research and development expenses for the year ended December 31, 2021 increased $44.9 million as compared to the prior year primarily due to an increase in research and clinical trial costs related to our ophthalmology, neurology, gene regulation and salivary gland programs, manufacturing of our clinical trial materials, payroll and payroll related costs, depreciation, rent and facility costs, share-based compensation and other research costs, which was partially offset by a decrease in acquired research and development costs.

Reimbursements under the Janssen collaboration agreement for the year ended December 31, 2021 increased $11.6 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, 2021 increased $0.1 million as compared to the prior year primarily due to the increase in allowable research and development costs.

Foreign currency loss was $6.3 million for the year ended December 31, 2021 compared to a gain of $3.4 million for the year ended December 31, 2020. The change of $9.7 million was primarily due to a weakening of the pound sterling and euro against the U.S. dollar and an increase in the amounts due from foreign subsidiaries in 2021.

Interest income was $0.2 million for the year ended December 31, 2021 compared to $1.3 million for the year ended December 31, 2020. The decrease was due to a lower average cash balance and lower interest rates during 2021.

Net loss attributable to ordinary shareholders for the year ended December 31, 2021 was $79.6 million, or $1.80 basic and diluted net loss per ordinary share, compared to a net loss attributable to ordinary shareholders of $58.0 million, or $1.54 basic and diluted net loss per ordinary share for the year ended December 31, 2020.

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 tight, dose responsive control of gene expression by oral small molecules with dynamic range that may exceed 5,000-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, including inherited retinal diseases and 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," "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, 2021, 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
(in thousands, except share and per share amounts)

	For the Years Ended December 31,	
	2021	2020
License revenue - related party	$ 37,701	$ 15,563
Operating expenses:		
General and administrative	43,765	44,207
Research and development	67,128	33,910
Total operating expenses	110,893	78,117
Loss from operations	(73,192)	(62,554)
Other non-operating income (expense):		
Foreign currency (loss) gain	(6,293)	3,426
Interest income	212	1,275
Interest expense	(288)	(139)
Net loss	(79,561)	(57,992)
Other comprehensive income (loss):		

Foreign currency translation gain (loss)	2,226	(3,103)
Total comprehensive loss	$ (77,335)	$ (61,095)
Net loss	$ (79,561)	$ (57,992)
Basic and diluted net loss per ordinary share	$ (1.80)	$ (1.54)
Weighted-average number of ordinary shares outstanding	44,139,655	37,724,189

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

	December31, 2021	December31, 2020
ASSETS		
CURRENT ASSETS:		
Cash and cash equivalents	$ 137,703	$ 209,520
Accounts receivable - related party	22,384	38,479
Prepaid expenses	8,102	7,082
Tax incentive receivable	12,634	12,930
Other current assets	2,420	4,565
Total Current Assets	183,243	272,576
Property, plant and equipment, net	75,860	44,042
Intangible assets, net	1,791	2,119
In-process research and development	783	852
Other assets	1,404	1,026
Equity method and other investments	6,656	—
Right-of-use assets - operating leases, net	22,782	21,486
Right-of-use assets - finance leases, net	27,645	21,596
TOTAL ASSETS	$ 320,164	$ 363,697
LIABILITIES AND SHAREHOLDERS' EQUITY		
CURRENT LIABILITIES:		
Accounts payable	$ 15,348	$ 7,134
Accrued expenses	27,586	20,861
Lease obligations, current	3,374	2,583
Deferred revenue - related party, current	21,820	23,545
Other current liabilities	—	24
Total Current Liabilities	68,128	54,147
Deferred revenue - related party	43,046	49,297
Lease obligations	20,359	19,666
Asset retirement obligations	2,081	1,814
Deferred income tax liability	196	214
Other long-term liabilities	953	—
TOTAL LIABILITIES	134,763	125,138
COMMITMENTS		
SHAREHOLDERS' EQUITY:		
Ordinary Shares, $0.00003881 par value, 1,288,327,750 authorized, 44,548,925 and 44,189,150 shares issued and outstanding at December 31, 2021 and 2020, respectively	2	2
Capital in excess of par value	528,659	504,482
Accumulated other comprehensive loss	(2,671)	(4,897)
Accumulated deficit	(340,589)	(261,028)
Total Shareholders' Equity	185,401	238,559
TOTAL LIABILITIES AND SHAREHOLDERS' EQUITY	$ 320,164	$ 363,697