



## MeiraGTx Reports First Quarter 2019 Financial Results and Provides Corporate Update

May 14, 2019

- Positive 6-month results from Phase 1/2 clinical trial of AAV-RPE65
- Enrollment completed in the Phase 1/2 clinical trial investigating AAV-CNGB3 gene therapy for the treatment of achromatopsia

LONDON and NEW YORK, May 14, 2019 (GLOBE NEWSWIRE) -- MeiraGTx Holdings plc (NASDAQ:MGTX), a vertically integrated, clinical stage gene therapy company, today announced financial results for the quarter ended March 31, 2019 and provided a corporate update.

MeiraGTx today reported positive topline data from the Company's Phase 1/2 trial of AAV-RPE65 for the treatment of patients with RPE65-deficiency. Additionally, MeiraGTx has completed dosing patients in its Phase 1/2 trial of AAV-CNGB3, an investigational gene therapy for the treatment of achromatopsia.

During the first quarter, MeiraGTx entered into a strategic collaboration with Janssen Pharmaceuticals, Inc. (Janssen), one of the Janssen Pharmaceutical Companies of Johnson & Johnson, to develop and commercialize gene therapies for the treatment of inherited retinal diseases (IRDs). This partnership has strengthened the Company's balance sheet and provided collaborative expertise from Janssen as MeiraGTx advances its broad portfolio of gene therapy candidates.

Manufacturing to support all of MeiraGTx's clinical programs is currently ongoing in the Company's wholly owned cGMP manufacturing facility in London.

"Thanks to the commitment of the MeiraGTx team, our global clinical partners and the patient community, we have made considerable progress advancing our pipeline of novel gene therapies through clinical development," said Alexandria Forbes, Ph.D., president and CEO. "We reached an important milestone with results from our first clinical trial, which showed RPE65-deficient patients gained significant improvements in vision, and we have also completed treating patients in our CNGB3 achromatopsia study."

### Recent Clinical Development Highlights

**Phase 1/2 trial of AAV-RPE65 for RPE65-Deficiency:** Today, MeiraGTx announced positive data from the Phase 1/2 dose finding trial of AAV-RPE65, the Company's investigational gene therapy for the treatment of RPE65-deficiency. A total of 15 patients were treated: nine adults in three dose escalation cohorts and six pediatric patients in an expansion cohort.

The trial achieved the primary endpoint of safety and tolerability of AAV-RPE65. Additionally, AAV-RPE65 demonstrated statistically significant improvement across several secondary endpoints designed to assess clinical activity.

MeiraGTx expects to meet with global regulatory authorities in the second half of 2019 to define the development pathway for regulatory approval. The company expects to present full data from the trial in a scientific forum later this year.

**Phase 1/2 trial of AAV-CNGB3 for Achromatopsia:** Enrollment in the study is now complete. A total of 23 patients were treated: 11 adults in dose escalation cohorts and 12 children in a pediatric expansion cohort.

**Phase 1/2 trial of AAV-RPGR for X-Linked Retinitis Pigmentosa (XLRP):** MeiraGTx is currently enrolling patients in the U.S. and UK in the randomized extension portion of the study.

**AAV-GAD for Parkinson's Disease:** MeiraGTx expects to meet with the FDA in mid-2019 to define the clinical pathway to support regulatory approval of AAV-GAD in Parkinson's disease. The Company anticipates providing a regulatory and clinical development update in the second half of 2019 following interactions with regulators.

**AAV-AQP1 for Grade 2/3 Radiation-Induced Xerostomia:** The Company's single center, Phase 1 dose finding study of AAV-AQP1 in patients with radiation-induced xerostomia following treatment for head and neck cancer continues to enroll with seven patients now treated in the first three cohorts of this trial at the National Institutes of Health (NIH). MeiraGTx expects to initiate an additional multi-center Phase 1/2 trial of AAV-AQP1 in 2019.

### First Quarter 2019 Corporate Highlights

**Entered into strategic collaboration with Janssen:** In January 2019, MeiraGTx entered into a strategic collaboration and licensing agreement with Janssen to develop and commercialize gene therapies for the treatment of IRDs.

**Strengthened balance sheet:** In March 2019, MeiraGTx raised approximately \$80 million of gross proceeds in a private placement of approximately 5.8 million of its ordinary shares. Johnson & Johnson Innovation —JJDC, Inc., the investment arm of Johnson & Johnson, and additional institutional investors participated in the offering.

**Added General Counsel to executive management team:** In April 2019, the Company strengthened its executive management team with the addition of Bruce Gottlieb as General Counsel and Corporate Secretary. Mr. Gottlieb brings significant regulatory and healthcare legal expertise to MeiraGTx.

**Appointment of new Directors:** In February and May 2019, MeiraGTx appointed Martin Indyk, Ph.D., and Nicole Seligman, respectively, to the Company's Board of Directors.

## First Quarter 2019 Financial Results

As of March 31, 2019, MeiraGTx had cash and cash equivalents of approximately \$227 million. In the first quarter of 2019, the company received approximately \$80 million of gross proceeds from a private placement of ordinary shares to institutional investors and an additional \$100 million from a collaboration, option and license agreement with Janssen. MeiraGTx believes this capital will be sufficient to fund its operating expenses and capital expenditure requirements into 2022.

## Comparison of Three Months Ended March 31, 2019 and 2018

General and administrative expenses were \$8.5 million for the three months ended March 31, 2019, compared to \$11.1 million for the three months ended March 31, 2018. The decrease of \$2.6 million was primarily due to decreases in payroll and share-based compensation, which was partially offset by increases in legal, insurance, travel expenses, investor relations and other general and administrative expenses.

Research and development expenses for the three months ended March 31, 2019 were \$13.0 million, compared to \$7.0 million for the three months ended March 31, 2018. The increase of \$6.0 million was primarily due to an increase in costs related to the amendment of our license agreement with UCL Business Plc (UCLB), our manufacturing facility, our clinical trials, consultants and legal fees, which was partially offset by \$1.3 million in research funding provided by our collaboration and license agreements with Janssen.

Foreign currency gain was \$2.7 million for the three months ended March 31, 2019 compared to a gain of \$1.0 million for the three months ended March 31, 2018. The increase of \$1.7 million was primarily due to a weakening of the U.S. dollar against the pound sterling during the three months ended March 31, 2019.

Net loss for the three months ended March 31, 2019 was \$18.0 million, or \$(0.62) basic and diluted net loss per ordinary share, compared to a net loss of \$16.4 million, or \$(1.91) basic and diluted net loss per ordinary share for the three months ended March 31, 2018.

MeiraGTx ended the first quarter of 2019 with \$227.3 million in cash and cash equivalents, compared to \$32.4 million as of March 31, 2018.

## About MeiraGTx

MeiraGTx (NASDAQ:MGTX) is a vertically integrated, clinical stage gene therapy company with five 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: inherited retinal diseases, neurodegenerative diseases and severe forms of xerostomia and xerophthalmia. 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](http://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 2019 milestones regarding its pre-clinical and clinical data and reporting of such data, meetings with regulatory authorities regarding pathways for regulatory approval of our product candidates, timing and results of data from the Phase 1/2 trial of AAV-RPE65 and timing of the initiation of trials in respect to its product candidates, 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, acquire additional capital, identify additional and develop existing product candidates, continue operating as a going concern, successfully execute strategic priorities, bring product candidates to market, build-out the manufacturing facility 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; 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; 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; litigation risks; and the other important factors discussed under the caption "Risk Factors" in our Annual Report on Form 10-K for the fiscal year ended December 31, 2018 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](http://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 BALANCE SHEETS  
 (unaudited)**

	<b>March 31, 2019</b>	<b>December 31, 2018</b>
<b>ASSETS</b>		
<b>CURRENT ASSETS:</b>		
Cash and cash equivalents	\$ 227,275,139	\$ 68,080,175
Prepaid expenses	1,765,209	1,937,785
Other current assets	2,117,334	4,634,105
<b>Total Current Assets</b>	<b>231,157,682</b>	<b>74,652,065</b>
Property and equipment, net	15,858,667	22,014,237
Security deposits	107,593	105,085
Restricted cash	123,376	123,376
Right-of-use assets	10,606,985	-
	<b>\$ 257,854,303</b>	<b>\$ 96,894,763</b>
<b>TOTAL ASSETS</b>		
<b>LIABILITIES AND SHAREHOLDERS' EQUITY</b>		
<b>CURRENT LIABILITIES:</b>		
Accounts payable	\$ 3,382,373	\$ 3,042,861
Accrued expenses	7,996,329	11,991,697
Lease obligations, current	679,025	27,199
Deferred revenue, related party, current	20,951,000	—
Other current liabilities	195,618	437,053
<b>Total Current Liabilities</b>	<b>33,204,345</b>	<b>15,498,810</b>
Deferred revenue - related party	77,186,561	—
Lease obligations	2,911,331	7,097
Deferred rent	-	201,264
Asset retirement obligations	133,816	128,119
<b>TOTAL LIABILITIES</b>	<b>113,436,053</b>	<b>15,835,290</b>
<b>COMMITMENTS</b>		
<b>SHAREHOLDERS' EQUITY:</b>		

Ordinary Shares, \$0.00003881 nominal value, 1,288,327,750 authorized 33,342,566 issued and outstanding at March 31, 2019 27,386,632 issued and outstanding at December 31, 2018	1,295	1,064
Capital in excess of nominal value	311,528,607	229,054,460
Accumulated other comprehensive (loss) income	(840,017 )	293,666
Accumulated deficit	(166,271,635 )	(148,289,717 )
Total Shareholders' Equity	144,418,250	81,059,473
TOTAL LIABILITIES AND SHAREHOLDERS' EQUITY	\$ 257,854,303	\$ 96,894,763

**MEIRAGTX HOLDINGS PLC AND SUBSIDIARIES**  
**CONDENSED CONSOLIDATED STATEMENTS OF OPERATIONS AND COMPREHENSIVE LOSS**  
**(unaudited)**

	For the Three Month Period Ended March 31,	
	2019	2018
	\$	\$
License revenue - related party	\$ 784,960	\$ -
Operating expenses:		
General and administrative	8,499,475	11,122,016
Research and development	12,976,229	6,927,322
Total operating expenses	21,475,704	18,049,338
Loss from operations	(20,690,744 )	(18,049,338 )
Other non-operating income (expense):		
Foreign currency gain	2,718,400	978,624
Change in fair value of warrant liability	-	669,408
Interest income	-	25,308
Interest expense	(9,574 )	(27,355 )
Net loss	(17,981,918 )	(16,403,353 )
Other comprehensive (loss):		
Foreign currency translation	(1,133,683 )	(757,765 )
Total comprehensive loss	\$ (19,115,601 )	\$ (17,161,118 )
Net loss	\$ (17,981,918 )	\$ (16,403,353 )
Accretion on convertible preferred C shares and warrants	-	(664,718 )
Adjusted net loss	\$ (17,981,918 )	\$ (17,068,071 )
Basic and diluted adjusted net loss per ordinary share	\$ (0.62 )	\$ (1.91 )
Weighted-average number of ordinary shares outstanding	28,776,915	8,927,433



Source: MeiraGTx