



## MeiraGTx Enters into Strategic Collaboration with Janssen to Develop and Commercialize Gene Therapy Treatments for Inherited Retinal Diseases

January 31, 2019

-- Conference call and webcast today, January 31, 2019 at 8:30 a.m. EST

-- MeiraGTx to receive \$100 million cash upfront payment upon closing

-- MeiraGTx to receive 20% net sales royalties and additional potential future milestone payments

-- Janssen Pharmaceuticals, Inc. to fund all clinical development and commercialization costs of MeiraGTx inherited retinal disease gene therapy programs

-- Janssen Pharmaceuticals, Inc. to receive worldwide exclusive rights to commercialize product candidates for achromatopsia (ACHM) caused by mutations in either CNGB3 or CNGA3, X-linked retinitis pigmentosa (XLRP) and options to additional IRD programs

LONDON and NEW YORK, Jan. 31, 2019 (GLOBE NEWSWIRE) -- MeiraGTx Holdings plc (NASDAQ:MGTX), a vertically integrated, clinical stage gene therapy company, today announced that it has entered into a broad 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).

Under the agreement, the two companies will collaborate in the clinical development of MeiraGTx's leading IRD pipeline, including product candidates for achromatopsia (ACHM) caused by mutations in either CNGB3 or CNGA3 and X-linked retinitis pigmentosa (XLRP). In addition, MeiraGTx and Janssen are entering into a research collaboration covering MeiraGTx's pipeline of pre-clinical programs for IRDs. The two companies are also entering into a research collaboration to further develop AAV manufacturing technology as well as clinical and commercial manufacturing supply agreements for the clinical and research programs.

"We are very excited to collaborate with Janssen, a leader in the world of innovation and the creation of new medicines. By combining Janssen's extensive clinical, regulatory and commercial expertise and global reach with MeiraGTx's deep experience in gene therapy development and manufacturing, we aim to accelerate the development of our pipeline of potential IRD gene therapies to address the needs of patients globally," said Alexandria Forbes, Ph.D., president and chief executive officer of MeiraGTx. "This important collaboration supports our leading position in gene therapy development and manufacturing and reaffirms our commitment to advancing a broad portfolio of breakthrough gene therapies that may improve the lives of patients suffering from IRDs worldwide."

### Details of the Agreement

Under the terms of the agreement, the two companies will collaborate in the clinical development of MeiraGTx's leading IRD pipeline, including product candidates for achromatopsia (ACHM) caused by mutations in either CNGB3 or CNGA3 and X-linked retinitis pigmentosa (XLRP). MeiraGTx will receive \$100 million in cash as an upfront payment and is eligible to receive additional payments for achieving development and sales milestones related to its CNGB3, CNGA3 and XLRP programs of up to \$340 million, and untiered royalties of 20 percent of annual net sales for the CNGB3, CNGA3 and XLRP programs. Janssen has agreed to fund all clinical development and commercialization costs for these programs.

MeiraGTx and Janssen are also entering into a research collaboration to develop a pipeline of IRD gene therapy candidates; a significant portion of the costs of the research collaboration will be paid by Janssen. Janssen has the right to opt-in to programs coming out of this research collaboration at the time of clearance of an Investigational New Drug (IND) application by the United States Food and Drug Administration (FDA). Janssen will fund all clinical development and commercialization costs following opt-in. MeiraGTx will receive an opt-in payment, development milestones and a high teens untiered royalty on annual net sales of commercialized products coming out of this collaboration.

The two companies are also entering into a research collaboration to further develop AAV manufacturing technology and will share the costs of this research collaboration.

This is MeiraGTx's second collaboration with Janssen, having entered into a research collaboration in October 2018 to develop regulatable gene therapy treatments using MeiraGTx's proprietary riboswitch technology.

IRDs are a group of rare eye conditions caused by an inherited gene mutation that are often characterized by progressive retinal degeneration which leads to severe vision impairment, loss or blindness. MeiraGTx currently has three ongoing clinical programs in IRDs, with a fourth program expected to enter clinical development in 1H2019, as well as a deep pipeline of pre-clinical IRD programs for a variety of gene mutations.

The transaction is subject to customary closing conditions, including the expiration of the applicable waiting period under the Hart-Scott-Rodino Antitrust Improvements Act of 1976 in the United States, and is expected to close in the first calendar quarter of 2019.

### Conference Call and Webcast Details

MeiraGTx will host a live conference call and webcast today, January 31, 2019, at 8:30 a.m. EST. The live webcast can be accessed by visiting the Investors section of the Company's website at [investors.meiragtx.com](http://investors.meiragtx.com). Please connect at least 15 minutes prior to the live webcast to ensure adequate time for any software download that may be needed to access the webcast. Alternatively, please call 1 (866) 796-1272 (U.S.) or 1 (409) 937-8924 (International) to listen to the live conference call. The conference ID number for the live call is 1398571. A replay of the webcast will be available on the Company's website for 30 days following the live conference call.

## 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, severe forms of xerostomia and neurodegenerative diseases. Though initially focusing on the eye, salivary gland and central nervous system, 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 Statements

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 the success of the research to be performed under the collaboration agreement, the development of our leading IRD product candidates and the development of our AAV manufacturing technology, 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 Quarterly Report on Form 10-Q for the quarterly period ended September 30, 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.

## Contacts

Investors:  
MeiraGTx  
Elizabeth Broder  
(646) 860-7983  
[investors@meiragtx.com](mailto:investors@meiragtx.com)

or

Media:  
W2O pure  
Christiana Pascale  
(212) 267-6722  
[cpascale@purecommunications.com](mailto:cpascale@purecommunications.com)



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