MeiraGTx Announces Exclusive Licensing Agreement with National Institutes of Health for Gene Therapy Treatment for Sjögren's Syndrome and Associated Xerostomia or Xerophthalmia

October 31, 2018

LONDON and NEW YORK, Oct. 31, 2018 (GLOBE NEWSWIRE) -- MeiraGTx Holdings plc (NASDAQ:MGTX), a vertically integrated, clinical stage gene therapy company, today announced an exclusive licensing agreement with the National Institute of Dental and Craniofacial Research (NIDCR), a division of the National Institutes of Health (NIH), an agency of the United States Department of Health & Human Services. Under the agreement, MeiraGTx will receive worldwide rights to adeno-associated virus vector mediated gene delivery of aquaporin-1, designated AAV-AQP1, for Sjögren's syndrome patients with associated xerostomia (dry mouth) or xerophthalmia (dry eyes). The license agreement includes standard and customary US Government terms and provisions. MeiraGTx and the NIDCR are currently partnered in two cooperative research and development agreements (CRADAs), one supporting preclinical work investigating AAV-AQP1 as a treatment for inadequate salivary gland function associated with Sjögren's syndrome, and the other covering an ongoing Phase 1 dose escalation clinical study of AAV-AQP1 in patients with grade 2 or 3 radiation-induced xerostomia.

Sjögren's syndrome is a systemic autoimmune disorder that impairs one's ability to secrete fluids such as saliva and tears. Symptoms include dry mouth, which can cause difficulty speaking, tasting food or swallowing, and dry eyes, which can be associated with itching, burning, blurry vision or intolerance to fluorescent lighting. Other serious symptoms can occur in Sjögren's syndrome, such as fatigue and chronic pain or damage to major organs.

"With as many as 4 million Americans living with Sjögren's syndrome, it is one of the most common autoimmune diseases, yet there are few therapeutic options. Sjogren's syndrome can be debilitating for patients and significantly impact their quality of life," said Alexandria Forbes, Ph.D., president and chief executive officer of MeiraGTx. "New treatment options for this disorder are urgently needed and we are very excited to continue working closely with the NIH on AAV-AQP1."

About Sjögren's Syndrome
Sjögren's syndrome is an autoimmune disorder which primarily attacks the glands that produce tears and saliva, impairing ability to secrete these fluids. The most common symptoms include dry eyes, dry mouth, fatigue, chronic pain and in severe cases result in damage to major organs. Sjögren's syndrome is often difficult to diagnose as characteristic features of this disorder can also be caused by many other conditions. It's estimated that Sjögren's syndrome affects more than 4 million Americans, and while the disorder can appear in both sexes and at all ages, Sjögren's syndrome is 10 times more prevalent in women than men. There is currently no cure for Sjögren's syndrome.

About MeiraGTx
MeiraGTx (NASDAQ:MGTX) is a vertically integrated, clinical stage gene therapy company with four ongoing clinical programs 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.

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 product pipeline, anticipated product benefits, goals and strategic priorities, product candidate development, growth expectations or targets and pre-clinical and clinical data, 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 June 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. 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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