MeiraGTx Presents Clinical Data on Botaretigene Sparoparvovec for the Treatment of X-Linked Retinitis Pigmentosa at the Association for Research in Vision and Ophthalmology 2022 Annual Meeting

May 04, 2022

LONDON and NEW YORK, May 04, 2022 (GLOBE NEWSWIRE) -- MeiraGTx Holdings plc (NASDAQ:MGTX), a vertically integrated, clinical-stage gene therapy company, today announced that additional clinical data from the Phase 1/2 trial of botaretigene sparoparvovec for the treatment of X-linked Retinitis Pigmentosa (XLRP) were presented at the Association for Research in Vision and Ophthalmology 2022 Annual Meeting (ARVO), in Denver, Colorado by Professor Michel Michaelides. A poster presentation of human retinal organoid vector efficacy data was also presented at the conference.

Oral Presentation Details:

Abstract Title: AAV5-RPGR (botaretigene sparoparvovec) Gene Therapy for X-linked Retinitis Pigmentosa (XLRP) Demonstrates Localized Improvements in Static Perimetry
Presenter: Dr. Michel Michaelides, Professor of Ophthalmology, UCL Institute of Ophthalmology in Dept. of Genetics
Date and Time: Wednesday, May 4, 2022, at 1:38 pm MDT (3:38 pm EDT)

Patients in the multicenter, open-label Phase 1/2 trial were given botaretigene sparoparvovec subretinally at 1 of 3 doses to the worse-seeing eye. Changes in retinal sensitivity and the volumetric analysis of the central 30 degrees of the retinal field were examined and compared to the untreated surrounding area as well as the retina of the untreated fellow eye. Exploration of the association between the location of botaretigene sparoparvovec delivery and changes in retinal sensitivity was undertaken by overlaying bleb topography onto sensitivity heat maps. Data from the study indicates improvements in photoreceptor function assessed through 12 months post-treatment suggesting local efficacy of botaretigene sparoparvovec gene therapy in XLRP patients. The treatment effect is observed within the treated bleb and may also extend beyond the margins of the bleb following surgery owing to subretinal extension before retinal reattachment.

Poster Presentation Details:

Abstract Title: AAV-RPGR Gene Therapy for *RPGR*-Associated X-Linked Retinitis Pigmentosa (XLRP): Human retinal organoid vector efficacy data
Presenting Author: Paul E. Sladen

Mutations within the retinitis pigmentosa GTPase regulator (*RPGR*) are the most frequent cause of (XLRP). This study investigated the efficacy of $RPGR^{ORF15}$ gene supplementation in human *RPGR*-deficient retinal organoids (ROs). Successful differentiation of RPGR-KO iPSCs was confirmed by qPCR and immunocytochemistry of major retinal and phototransduction markers. Viral transduction of RPGR-KO ROs with AAV-RPGR led to restoration of *RPGR* expression in human rods and cones. *RPGR* was localized at the photoreceptor cilium and led to marked improvements in several molecular, phenotypic readouts. The *RPGR* transgene was correctly expressed, processed, and localized in human rods and cones following viral transduction of *RPGR*-deficient human ROs. These data are consistent with the reported Phase 1/2 trial positive results for botaretigene sparoparvovec in patients with *RPGR*-associated XLRP.

MeiraGTx and Janssen Pharmaceuticals, Inc., part of the Janssen Pharmaceutical Companies of Johnson & Johnson, are jointly developing botaretigene sparoparvovec as part of a broader collaboration to develop and commercialize gene therapies for the treatment of inherited retinal diseases. MeiraGTx remains eligible to receive additional development and commercial milestones for botaretigene sparoparvovec as well as for other programs as part of the collaboration agreement.

About Botaretigene Sparoparvovec
Botaretigene sparoparvovec, formerly referred to as AAV-RPGR, is an investigational gene therapy for the treatment of patients with XLRP caused by disease-causing variants in the eye-specific form of the *RPGR* gene (RPGR ORF15). Botaretigene sparoparvovec is designed to deliver functional copies of the *RPGR* gene to the subretinal space in order to help improve and/or preserve vision. Botaretigene sparoparvovec is being evaluated in the Phase 1/2 MGT009 clinical trial (NCT03252847), an open-label, multi-center dose escalation study with a randomized, multi-dose expansion cohort which included a deferred treatment control to determine the safety and efficacy of botaretigene sparoparvovec in adults and children with XLRP caused by variants in the *RPGR* gene. The Lumeos trial (NCT04671433), a Phase 3 randomized, controlled study of botaretigene sparoparvovec for the treatment of XLRP associated with variants in the *RPGR* gene, is actively dosing patients. Botaretigene sparoparvovec has been granted Fast Track and Orphan Drug designations by the FDA and PRIority MEdicines (PRIME), Advanced Therapy Medicinal Product (ATMP) and Orphan designations by the European Medicines Agency (EMA).

About X-Linked Retinitis Pigmentosa (XLRP)
XLRP is the most severe form of retinitis pigmentosa (RP), a group of inherited retinal diseases characterized by progressive retinal degeneration and vision loss. In XLRP, both rods and cones function poorly, leading to a degeneration of the retina and total blindness. The most frequent cause of XLRP is disease-causing variants in the *RPGR* gene, accounting for more than 70% of cases of XLRP, and up to 20% of all cases of RP. There are currently no approved treatments for XLRP.

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

a dynamic range that can exceed 5000-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 the development and efficacy of botaretigene sparoparvovec, the Phase 3 Lumeos clinical trial of botaretigene sparoparvovec and the achievement of milestones or regulatory approvals, including in light of the COVID-19 pandemic, as well as statements that include the words "expect," "will," "intend," "plan," "believe," "project," "forecast," "estimate," "may," "could," "should," "would," "continue," "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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