MeiraGTx Announces Upcoming Presentations at the Association for Research in Vision and Ophthalmology (ARVO) 2023 Annual Meeting

April 21, 2023

LONDON and NEW YORK, April 21, 2023 (GLOBE NEWSWIRE) -- MeiraGTx Holdings plc (Nasdaq: MGTX), a vertically integrated, clinical stage gene therapy company, today announced two abstract presentations at the Association for Research in Vision and Ophthalmology (ARVO) 2023 Annual Meeting.

The first abstract will present humoral immune response data from the Company's Phase 1/2 MGT009 trial for the investigational gene therapy botaretigene sparoparvovec (bota-vec, formerly AAV-RPGR) in patients with the inherited retinal disease (IRD) X-linked retinitis pigmentosa (XLRP) associated with the retinitis pigmentosa GTPase regulator (RPGR) gene which is being developed along with our partners Janssen Pharmaceuticals, Inc. (Janssen), one of the Janssen Pharmaceutical Companies of Johnson & Johnson.

The second abstract will present data from MeiraGTx's proprietary promoter platform involving the use of novel, AI-assisted engineered promoters to improve gene expression in rod photoreceptors.

"We are pleased to present recent data from the Phase 1/2 clinical study of our investigational gene therapy bota-vec at this year's ARVO meeting. An understanding of the immune response of bota-vec in XLRP patients is important as we move forward in the late stage development of this product with the aim of providing a safe and effective treatment for patients with this irreversible and devastating disease," said Alexandria Forbes, Ph.D., president, and chief executive officer of MeiraGTx. "In addition, I am very excited to present some of the first data from our AI driven promoter discovery platform as exemplified by the rod-specific promoters we are presenting. Our AI driven promoter discovery platform provides us the ability to rapidly and accurately achieve cell specific as well as gene-appropriate expression levels in any cell type, accelerating the development of optimized potent and safe gene therapy vectors targeting different cell types and tissues, including different neuronal sub-types. The rod specific promoters presented are being applied to our optogenetic vectors targeting rods to allow these photoreceptors to respond more like cones, a potential treatment for central vision loss in diseases such as Dry AMD, as well as for rod or rod-cone disorders."

Presentation/poster #:

5446: Humoral immune response to AAV5-RPGR (botaretigene sparoparvovec) gene therapy in RPGR-associated X-linked retinitis pigmentosa

- April 27, 2023, 2:15-2:30pm CDT
- Session #300, Location - 353-355

CO405: AAV-based evaluation of novel in silico promoters to drive expression in rod photoreceptors

- April 23, 2023, 12:00-1:45 pm CDT
- Session #49, Location – Exhibit Hall

The presentations will be available on the publications page of the Company's website after their respective presentation times. The abstracts can be found on the ARVO Annual Meeting website.

MeiraGTx and Janssen are jointly developing bota-vec as part of a broader collaboration to develop and commercialize gene therapies for the treatment of inherited retinal diseases.

About botaretigene sparoparvovec (bota-vec)
Botaretigene sparoparvovec (bota-vec) is being investigated in collaboration with Janssen for the treatment of patients with XLRP caused by disease-causing variants in the eye-specific form of the *RPGR* gene. Through a one-time administration, bota-vec is designed to deliver functional copies of the *RPGR* gene to counteract the loss of retinal cells with the goal of preserving and potentially restoring vision for those living with XLRP. The Phase 3 LUMEOS clinical trial (NCT04671433) is actively dosing patients to study bota-vec for the treatment of patients with XLRP with disease-causing variants in the *RPGR* gene. Bota-vec has been granted Fast Track and Orphan Drug designations by the U.S. Food and Drug Administration (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 a rare condition estimated to impact one in 40,000 people globally.[1,2] People with XLRP have progressive vision loss, starting in childhood with night blindness.[3] Over time, they lose their peripheral vision leading to central vision loss followed by legal blindness by the fourth decade of life.[3] Currently, there are no approved treatments for XLRP.[3]

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 that allows precise, dose responsive control of gene expression by oral small molecules with 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 both inherited retinal diseases as well as 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.

Contacts

Investors:
MeiraGTx
Investors@meiragtx.com

Media:
Jason Braco, Ph.D.
LifeSci Communications
jbraco@lifescicomms.com

[1] Boughman JA, Conneally PM, Nance WE. Population genetic studies of retinitis pigmentosa. Am J Hum Genet. 1980;32(2):223–235.
[2] Fishman GA. Retinitis pigmentosa. Genetic percentages. Arch Ophthalmol. 1978;96(5):822–826. doi:10.1001/archopht.1978.03910050428005.
[3] Wang DY, Chan WM, Tam PO, et al. Gene mutations in retinitis pigmentosa and their clinical implications. Clin Chim Acta. 2005;351(1-2):5-16.