MeiraGTx Receives Rare Pediatric Disease Designation from FDA for AAV8-RK-RetGC for the Treatment of Patients with Leber Congenital Amaurosis due to GUCY2D Mutations

January 22, 2025

MeiraGTx has recently received Rare Pediatric Disease Designation (RPDD) for four inherited retinal diseases (IRDs) reflecting the transformative therapeutic potential of the Company's proprietary technology platforms

LONDON and NEW YORK, Jan. 22, 2025 (GLOBE NEWSWIRE) -- MeiraGTx Holdings Plc (Nasdaq: MGTX), a vertically integrated, clinical-stage genetic medicines company, today announced the U.S. Food and Drug Administration (FDA) has granted the Company Rare Pediatric Disease Designation to its AAV8-RK-RetGC program for the treatment of patients with Leber congenital amaurosis due to *GUCY2D* mutations (LCA1). This is the fourth Rare Pediatric Disease Designation the Company has received in the last three months, including AAV8-RK-AIPL1 for the treatment of LCA4 retinal dystrophy, AAV8-RK-BBS10 for the treatment of Bardet-Biedl syndrome (BBS) due to *BBS10* mutations and AAV5-RDH12 for the treatment of *RDH12* associated retinal dystrophy.

"Receiving Rare Pediatric Disease Designation for an additional program in our ophthalmology pipeline represents another regulatory milestone for the Company and demonstrates the groundbreaking therapeutic potential of our technology to address these severe childhood blinding conditions," said Alexandria Forbes, Ph.D., president and chief executive officer of MeiraGTx.

"As we have done with our AIPL1 program, we intend to leverage our manufacturing infrastructure and Specials License along with our clinical expertise in IRDs to work with regulators to expedite the delivery of these potentially life changing treatments to these severely affected children."

An RPDD may be granted by the FDA to drugs and biologics intended to treat certain orphan diseases affecting fewer than 200,000 patients in the U.S., the serious or life-threatening manifestations of which primarily affect individuals aged 18 years or younger. Under the FDA's Rare Pediatric Disease Priority Review Voucher (PRV) program, a sponsor that receives approval for a biologics license application for a rare pediatric disease may be eligible to receive a voucher for a priority review of a subsequent marketing application for a different product. PRVs may be used by the sponsor or sold to another sponsor for their use and have recently sold for between $100 million to $158 million.

About AAV8-RK-RetGC

Mutations in the *GUCY2D* gene coding for guanylate cyclase lead to severe retinal diseases in humans, with 88% of cases causing autosomal recessive Leber congenital amaurosis type 1 (LCA1) whilst heterozygous missense mutations cause autosomal dominant cone-rod dystrophy (CRD). In LCA1, photoreceptor function loss and blindness emerge very early in life. In CRD, degeneration starts in the cones and leads to loss of the central visual field due to the high presence of cones in the macula. CRD can lead to complete blindness when degeneration of rods follows those of cones.

About AAV8-RK-AIPL1

AAV8-RK-AIPL1 is an investigational genetic medicine for the treatment of one of the most severe forms of Leber congenital amaurosis (LCA) owing to genetic deficiency of Aryl-hydrocarbon-interacting protein-like 1 (AIPL1). It is delivered via subretinal injection to children, and through a one-time administration, AAV8-RK-AIPL1 is designed to deliver functional copies of the AIPL1 gene to cone and rod photoreceptors in the central retina, to slow further degeneration and restore vision.

About AAV8-RK-BBS10

The investigational genetic medicine AAV8-RK-BBS10 is an adeno-associated virus with a serotype 8 capsid with a complementary DNA (cDNA) encoding the human *BBS10* gene for treatment of Bardet-Biedl syndrome (BBS) due to *BBS10* mutations. BBS is a rare genetic disease affecting approximately 1 in 250,000 people around the world. One of the primary symptoms of BBS is visual impairment secondary to retinal degeneration. More than 20 different genes are associated with the development of BBS, with BBS10 accounting for approximately 25% of cases.

About AAV5-RDH12

The investigational genetic medicine AAV5-RDH12 is an adeno-associated virus serotype 5 containing the human RDH12 gene for treatment of RDH12 associated retinal dystrophy. Defects in retinol dehydrogenase 12 (RDH12) account for 3–10% of Leber congenital amaurosis (LCA) and early-onset severe retinal dystrophy (EOSRD) and is particularly devastating due to early macular atrophy. RDH12 encodes retinol dehydrogenase 12, an enzyme expressed in photoreceptors that reduces all-trans-retinal to all-trans-retinol.

About MeiraGTx

MeiraGTx (Nasdaq: MGTX) is a vertically integrated, clinical-stage genetic medicines company with a broad pipeline of late-stage clinical programs supported by end-to-end manufacturing capabilities. MeiraGTx has internal plasmid production for GMP, two GMP viral vector production facilities as well as an in-house Quality Control hub for stability and release, all fit for IND through commercial supply. In addition, MeiraGTx has developed a proprietary manufacturing platform with leading yield and quality aspects and commercial readiness, core capabilities in viral vector design and optimization and a transformative riboswitch gene regulation platform technology that allows for the precise, dose-responsive control of gene expression by oral small molecules. MeiraGTx is focusing the riboswitch platform on the delivery of metabolic peptides, including GLP-1, GIP, Glucagon, and PYY, using oral small molecules, as well as cell therapy for oncology and autoimmune diseases. MeiraGTx has developed the technology to apply genetic medicine to more common diseases, increasing efficacy, addressing novel targets, and expanding access in some of the largest disease areas where the unmet need remains high.

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 our product candidate development and anticipated milestones regarding our pre-clinical and clinical data, reporting of such data and the timing of results of data and regulatory matters, 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, repay our debt obligations, identify additional and develop existing product candidates, successfully execute strategic transactions or 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 or rare pediatric disease designations, retain key personnel or attract qualified employees, or incur expected levels of operating expenses; the impact of pandemics, epidemics or outbreaks of infectious diseases 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 Quarterly Report on Form 10-Q for the quarter ended September 30, 2024, 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.

Contacts

Investors:
MeiraGTx
Investors@meiragtx.com

or

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