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Opus Genetics Announces Completion of Dosing in First Cohort of Phase 1/2 Trial of Gene Therapy OPGx-LCA5 in Patients with Rare Inherited Retinal Disease LCA5

OPGx-LCA5 will advance to the next highest dose in mid-2024 based on positive safety and efficacy data

OPGx-LCA5 is well-tolerated and demonstrated clear signs of biological activity

RESEARCH TRIANGLE PARK, N.C., March 26, 2024 (GLOBE NEWSWIRE) -- Opus Genetics, a patient-first, clinical-stage gene therapy company developing treatments for inherited retinal diseases, today announced that the first cohort has completed dosing in its open-label, dose-escalation Phase 1/2 clinical trial evaluating the subretinal delivery of OPGx-LCA5, an adeno-associated virus 8 (AAV8) vector designed to precisely deliver a functional *LCA5* gene to the outer retina in patients with Leber congenital amaurosis (LCA) resulting from biallelic mutations in the *LCA5* gene (*LCA5*).

Based on positive safety and efficacy data from the first cohort of three adult patients, the Company will advance OPGx-LCA5 into the next highest dose. Opus anticipates initiating the next cohort mid-2024. There are also future plans to expand the study population to include subjects 13 years or older.

“In the first cohort, OPGx-LCA5 has been well-tolerated and demonstrated clear signs of biological activity through 90 days, warranting continued evaluation in the next highest dose,” said principal investigator Tomas S. Aleman, M.D., from the Center for Advanced Retinal and Ocular Therapeutics (CAROT), of the Scheie Eye Institute, Department of Ophthalmology of the Perelman School of Medicine, University of Pennsylvania. “Moreover, early anecdotal and VR challenge test feedback is encouraging and indicates that some of the patients, who have been nearly totally blind all their lives, are now able to see and identify objects for the first time.”

LCA5 is a form of an early-onset retinal degeneration that affects approximately one in 1.7 million people in the U.S. Currently, there are no approved treatments for individuals with *LCA5*-related vision loss.

“Based on these early clinical data, we’re excited for the potential of OPGx-LCA5 to transform the lives of patients affected by *LCA5*,” said Ben Yerxa, Ph.D., chief executive officer of Opus. “We’d like to thank the study participants and their families and the incredible efforts of the team at the University of Pennsylvania for reaching this clinical

milestone, and we look forward to progressing the trial as we continue to dose escalate."

For more information on the trial, visit clinicaltrials.gov (NCT05616793).

About OPGx-LCA5
OPGx-LCA5 is designed to address a form of Leber congenital amaurosis (LCA) due to biallelic mutations in the *LCA5* gene (*LCA5*), which encodes the lebercilin protein. *LCA5* is an early-onset severe inherited retinal dystrophy. Studies in *LCA5* patients have reported evidence for the dissociation of retinal architecture and visual function in this disease, suggesting an opportunity for therapeutic intervention through gene augmentation. OPGx-LCA5 uses an adeno-associated virus 8 (AAV8) vector to precisely deliver a functional *LCA5* gene to the outer retina. OPGx-LCA5 is currently being evaluated in a Phase 1/2 clinical trial at the University of Pennsylvania designed to evaluate its safety and preliminary efficacy in nine patients with inherited retinal degeneration due to biallelic mutations in the LCA5 gene.

About Opus Genetics
Opus Genetics is a clinical-stage gene therapy company for inherited retinal diseases with a unique model and purpose. Backed by Foundation Fighting Blindness' venture arm, the RD Fund, Opus combines unparalleled insight and commitment to patient need with wholly owned programs in numerous orphan retinal diseases. Its AAV-based gene therapy portfolio, including a derisked *LCA5* lead program currently in a Phase 1/2 clinical trial, tackles some of the most neglected forms of inherited blindness while creating novel orphan manufacturing scale and efficiencies. Based in Research Triangle Park, N.C., the company leverages knowledge of the best science and the expertise of pioneers in ocular gene therapy to transparently drive transformative treatments to patients. For more information, visit www.opusgenetics.com.

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