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

OPGx-LCA5 is designed to address vision loss due to Leber congenital amaurosis associated with mutations in the LCA5 *gene, which causes one of the most severe early-onset retinal dystrophies*

Company's first product candidate from robust pipeline of gene therapies for inherited retinal diseases advances into clinic

RESEARCH TRIANGLE PARK, N.C., Sept. 07, 2023 (GLOBE NEWSWIRE) -- Opus Genetics, a patient-first, clinical-stage gene therapy company developing treatments for inherited retinal diseases, today announced that the first patient has been dosed in its Phase 1/2, first-in-human clinical trial 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*).

LCA5 is 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.

"Dosing our first patient establishes Opus as a clinical-stage company and is a point of progress in our mission to advance first-in-class gene therapies for inherited retinal diseases," said Ben Yerxa, Ph.D., chief executive officer of Opus. "Despite the severe retinal dysfunction in patients with *LCA5*, preclinical data suggest an opportunity for therapeutic intervention, including retinal structural and functional restoration when OPGx-LCA5 was administered prior to peak disease severity. We look forward to progressing the trial of this potentially transformative therapy for patients affected by *LCA5."*

The Phase 1/2, open-label, dose-escalation trial is evaluating the subretinal delivery of OPGx-LCA5 in nine adult patients with *LCA5*. The objective of the trial is to evaluate the safety and preliminary efficacy of OPGx-LCA5 in patients with inherited retinal degeneration due to biallelic mutations in the *LCA5* gene.

Once safety in adults has been established and confirmed by the FDA, Opus plans to add a pediatric cohort.

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. Preclinical data, including animal and human iPSC models, have demonstrated preservation of retinal structure and visual function when OPGx-LCA5 was administered prior to peak disease severity.

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 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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