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Opus Genetics Receives FDA Clearance of IND Application for OPGx-001, a Gene Therapy Candidate Intended for the Treatment of Rare Inherited Retinal Disease LCA5

OPGx-001 is Opus' first program to enter clinical evaluation and is designed to address vision loss due to mutations in the LCA5 *gene, which causes one of the most severe forms of early-onset blinding disease Leber congenital amaurosis*

Company anticipates initiating a Phase 1/2 clinical trial in early 2023 in the U.S.

RESEARCH TRIANGLE PARK, N.C., Dec. 01, 2022 (GLOBE NEWSWIRE) -- Opus Genetics, a patient-first, clinical-stage gene therapy company developing treatments for inherited retinal diseases, today announced the U.S. Food and Drug Administration (FDA) has cleared its Investigational New Drug (IND) application for a Phase 1/2, first-in-human clinical trial of OPGx-001 in patients with Leber congenital amaurosis (LCA) resulting from biallelic mutations in the *LCA5* gene (*LCA5*). OPGx-001 is an adeno-associated virus 8 (AAV8) vector to precisely deliver a functional *LCA5* gene to retinal photoreceptors. Currently, there are no approved treatments for individuals with *LCA5*-related vision loss.

"We founded Opus a little more than a year ago to quickly move promising potential treatments into the clinic for patients in need. This FDA clearance of our IND application for OPGx-001 for *LCA5* marks a significant milestone for Opus, as our first program to enter the clinic," said Ben Yerxa, Ph.D., Chief Executive Officer of Opus. "Preclinical studies in in vitro and in vivo models of *LCA5* have provided support for the safety and efficacy of OPGx-001. We look forward to initiating our first-in-human trial of OPGx-001 in early 2023 and to continuing to build and advance our pipeline of gene therapies for unaddressed inherited retinal diseases in parallel."

The Phase 1/2, open-label, dose-escalation trial will evaluate the subretinal delivery of OPGx-001 in nine adult patients with *LCA5*. The objective of the trial is to evaluate safety and potential benefit. Once safety in adults has been cleared, Opus plans to add a pediatric cohort.

For more information, visit clinicaltrials.gov (NCT05616793).

About OPGx-001
OPGx-001 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 degeneration. 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-001 utilizes an adeno-associated virus 8 (AAV8) vector to precisely deliver a functional *LCA5* gene to photoreceptors in the retina. Preclinical data, including animal and human iPSC models, have demonstrated preservation of retinal structure and visual function when OPGx-001 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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