August 20, 2024

Opus Genetics Receives Rare Pediatric Disease Designation from the U.S. FDA for Ocular Gene Therapy OPGx-LCA5 to Treat Rare Inherited Retinal Disease LCA5

Priority Review Voucher would be issued upon approval of OPGx-LCA5

RESEARCH TRIANGLE PARK, N.C., Aug. 20, 2024 (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 granted Rare Pediatric Disease designation (RPD) for its ocular gene therapy OPGx-LCA5 to treat patients with the inherited retinal disease *LCA5*. OPGx-LCA5 is 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*).

“We are thrilled to receive Rare Pediatric Disease designation from the FDA for our OPGx-LCA5 gene therapy. This important milestone brings us closer to delivering a potential treatment for patients with *LCA5*,” said Ben Yerxa, Ph.D., chief executive officer of Opus. “At Opus, we’re committed to advancing therapies that help treat patients with inherited retinal diseases, and this designation further validates the potential impact of our innovative ocular gene therapy approach. We look forward to providing updates on the Phase 1/2 clinical trial evaluating OPGx-LCA5 soon.”

LCA5 is a form of 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. OPGx-LCA5 is currently being studied in an open-label, dose-escalation Phase 1/2 clinical trial at the University of Pennsylvania designed to evaluate its safety and preliminary efficacy in 15 patients with inherited retinal degeneration due to biallelic mutations in the *LCA5* gene. For more information on the trial, visit clinicaltrials.gov (NCT05616793).

The FDA grants Rare Pediatric Disease designation to therapeutics intended to treat serious or life-threatening rare diseases that primarily affect individuals under the age of 18. By obtaining this designation, Opus has access to valuable incentives and support from the FDA during its development program. With this designation, OPGx-LCA5 will be eligible to receive a priority review voucher upon approval for any subsequent marketing application that can be sold or transferred to other companies.

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 15 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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Source: Opus Genetics