May 4, 2026

Opus Genetics Announces FDA Acceptance of OPGx-LCA5 into Rare Disease Evidence Principles (RDEP) Program

RESEARCH TRIANGLE PARK, N.C., May 04, 2026 (GLOBE NEWSWIRE) -- Opus Genetics, Inc. (Nasdaq: IRD) ("Opus Genetics" or the "Company"), a clinical-stage biopharmaceutical company developing gene therapies to restore vision and prevent blindness in patients with inherited retinal diseases (IRDs), today announced that its investigational LCA5 gene therapy program, OPGx-LCA5, has been accepted into the U.S. Food and Drug Administration's (FDA) Rare Disease Evidence Principles (RDEP) program.

OPGx-LCA5 is a potential gene therapy for Leber congenital amaurosis type 5 (LCA5), a rare inherited retinal disease caused by mutations in the LCA5 gene. The condition leads to early-onset, progressive vision loss and often results in severe visual impairment or blindness in childhood. There are currently no approved therapies specifically targeting LCA5.

RDEP is a new FDA initiative intended to support the development of therapies for ultra-rare genetic diseases typically affecting fewer than 1,000 patients in the U.S. The program enables early and ongoing collaboration between the FDA and sponsors to align on regulatory strategy, clinical trial design, and innovative approaches to generating evidence needed to support potential approval.

"RDEP eligibility represents an important element of our regulatory strategy as we seek alignment with the FDA on our pivotal Phase 3 program for OPGx-LCA5," said George Magrath, M.D., Chief Executive Officer, Opus Genetics. "Given the rarity and severity of this disease, early engagement with the FDA alongside our RMAT designation will help inform a more efficient and streamlined development pathway. We look forward to collaborating with the FDA as we pursue a potential treatment option for patients affected by this devastating inherited retinal disease."

As part of the RDEP program, the FDA will work closely with Opus Genetics to guide the ongoing development of OPGx-LCA5, including considerations for clinical trial design, approaches to generating efficacy in a small patient population, and strategies to support demonstration of clinical benefit. The program also provides a framework for evaluating substantial evidence of effectiveness, including the potential use of a single adequate and well-controlled study supported by confirmatory evidence.

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-associated inherited retinal disease is an early-onset severe inherited retinal dystrophy. Studies in patients with this mutation 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. Data from pediatric participants demonstrated large gains in cone-mediated vision, and the therapy remains well tolerated with no ocular serious adverse events or dose-limiting toxicities. The adult cohort showed durable improvements in cone sensitivity and visual function out to 18 months. OPGx-LCA5 has also received Rare Pediatric Disease, Orphan Drug, Regenerative Medicine Advanced Therapy (RMAT) designations from the FDA.

About Opus Genetics

Opus Genetics is a clinical-stage biopharmaceutical company developing gene therapies to restore vision and prevent blindness in patients with inherited retinal diseases (IRDs). The Company is developing durable, one-time treatments designed to address the underlying genetic causes of severe retinal disorders. The Company's pipeline includes seven AAV-based programs, led by OPGx-LCA5 for LCA5-related mutations and OPGx-BEST1 for BEST1-related retinal degeneration, with additional candidates targeting RDH12, MERTK, RHO, CNGB1 and NMNAT1. Opus Genetics is also advancing a small-molecule therapy, Phentolamine Ophthalmic Solution 0.75%, beyond its approved use for pharmacologically induced mydriasis, with a supplemental new drug application under review for presbyopia and an ongoing Phase 3 pivotal trial for mesopic, low contrast conditions after keratorefractive surgery (dim light disturbances). The Company is based in Research Triangle Park, NC. For more information, visit www.opusgtx.com.

Forward Looking Statements

This press release contains forward-looking statements within the meaning of the Private Securities Litigation Reform Act of 1995. Such statements include, but are not limited to, statements related to the clinical development, clinical results, preclinical data, and future plans for OPGx-LCA5 and expectations regarding us, our business prospects, and our results of operations and are subject to certain risks and uncertainties posed by many factors and events that could cause our actual business, prospects and results of operations to differ materially from those anticipated by such forward-looking statements. Factors that could cause or contribute to such differences include, but are not limited to, those described under the heading "Risk Factors" included in our Annual Report on Form 10-K for the fiscal year ended December 31, 2025, and in our other filings with the U.S. Securities and Exchange Commission. Readers are cautioned not to place undue reliance on these forward-looking statements, which speak only as of the date of this press release. These forward-looking statements are based upon our current expectations and involve assumptions that may never materialize or may prove to be incorrect. Actual results and the timing of events could differ materially from those anticipated in such forward-looking statements as a result of various risks and uncertainties. In some cases, you can identify forward-looking statements by the following words: "anticipate," "believe," "continue," "could," "estimate," "expect," "intend," "aim," "may," "ongoing," "plan," "potential," "predict," "project," "should," "strive," "will," "would" or the negative of these terms or other comparable terminology, although not all forward-looking statements contain these words. We undertake

no obligation to revise any forward-looking statements in order to reflect events or circumstances that might subsequently arise.

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