May 7, 2026

Opus Genetics Presents Clinical and Preclinical Data at ARVO 2026 Demonstrating Continued Pipeline Advancement in Inherited Retinal Diseases

Six-Month Clinical Data Demonstrate Restoration of Cone-Mediated Vision in Pediatric LCA5 Patients, with Sensitivity Improvements Reaching Normal Ranges

Early BEST1 Data Show Visual Acuity Gains and Improved Retinal Structure, Supporting Expansion into a Large IRD Population

Preclinical RHO Programs Demonstrate Durable Retinal Preservation and Support Clinical Translation

Mutation-Independent AAV Approach Highlights Potential to Address Genetically Diverse Retinal Diseases with a Single Therapy

Advancements In Potency Assays and Manufacturing Reinforce Scalability and Regulatory Readiness

RESEARCH TRIANGLE PARK, N.C., May 07, 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 new clinical and preclinical data from multiple programs presented at the Association for Research in Vision and Ophthalmology (ARVO) Annual Meeting 2026 in Denver, Colorado.

The data highlight emerging evidence that Opus Genetics’ gene therapy OPGx-LCA5 may restore daytime vision mediated by cones in pediatric patients with severe, early-onset disease, while also advancing Opus Genetics’ broader pipeline across BEST1 and RHO programs.

“Our ARVO presentations reflect a meaningful shift in what gene therapy can achieve in retinal disease,” said George Magrath, M.D., Chief Executive Officer, Opus Genetics. “We are seeing consistent evidence that we may be able to rapidly restore visual function, with encouraging clinical signals and a growing foundation across multiple programs for patients with genetic blinding diseases.”

Oral Presentation

Restoration of Cone-Mediated Vision After Gene Augmentation in Children with LCA5

The oral presentation, delivered by Tomas S. Aleman, M.D., primary study investigator, reported 6-month results from the ongoing Phase 1/2/3 study of OPGx-LCA5 in pediatric patients with Leber congenital amaurosis type 5 (LCA5), a severe, early-onset, inherited retinal degeneration.

Despite advanced disease and very poor baseline vision, patients treated with OPGx-LCA5 demonstrated robust and consistent restoration of cone-mediated function following a single subretinal injection.

Key findings include:

- Well tolerated, with no dose-limiting toxicities and adverse events that were mild, expected, and resolved
- Over 30-fold improvements in cone sensitivity across all treated patients, representing recovery of primary photoreceptor function
- Improvement in visual acuity in treated eyes relative to baseline and untreated control eyes
- Objective confirmation of efficacy across multiple independent readouts, including dark-adapted pupillary light responses showing improved amplitude and latency thresholds
- Early and durable improvements in functional vision in an orientation and mobility test
- Consistent patient-reported improvements in daily functional vision

“These results indicate that even in severe, early-onset disease, there remains a possible window of opportunity to restore cone function and meaningfully improve vision,” said Dr. Tomas S. Aleman. “Gene augmentation in LCA5 demonstrated dramatic recovery of daytime vision in adolescents, paving the path for use in younger patients with theoretically greater treatment potential. Importantly, these results build on prior adult data and demonstrate that in severe pediatric disease, viable cone photoreceptors may be rescued, supporting a broader therapeutic window.”

Poster Presentations

Preliminary Results from Adult Participant in a Phase 1b/2a Clinical Study of OPGx-BEST1 Gene Therapy for ARB and BVMD Due to BEST1 Mutations

- Well tolerated through three months with no ocular inflammation or treatment-related adverse events
- Up to 12-letter improvement in visual acuity in the treated eye
- ~23% reduction in central subfield thickness, indicating improved retinal structure
- Early patient-reported improvements, including reduced perception of progressive vision dimming

These findings provide early clinical evidence for BEST1 gene augmentation in a large inherited retinal disease population. Opus Genetics has completed enrollment in Cohort 1 of its ongoing Phase 1/2 study of OPGx-BEST1 gene therapy, and expects to announce 3-month topline data from Cohort 1 in September 2026.

Development of Cell-Based Expression and Functional Potency Assays for OPGx-BEST1 Gene Therapy

- Developed robust, reproducible assays (RT-dPCR, western blot, ELISA) to quantify gene expression and protein production
- Demonstrated consistent potency across manufacturing lots within expected ranges (50–150%)
- Established a functional assay to measure BEST1 channel activity, supporting mechanism-driven validation

These capabilities support scalable development and regulatory readiness.

Nonclinical Efficacy and Toxicity Study of GMP-Grade Vector OPGx-RHO Delivered by Subretinal Injection in a Canine Model of RHO-adRP

- Established no-observed-adverse-effect-level (NOAEL) to guide clinical dosing
- Demonstrated preservation of retinal structure and function in treated regions
- Observed dose-dependent structural rescue, supporting clinical translation

Therapeutic Efficacy of a Mutation-Independent AAV Knockdown and Replacement Approach in a Swine Animal Model of Autosomal-Dominant Retinitis Pigmentosa (RHO)

- Mutation-independent approach demonstrated:
  - Restoration of rod-driven visual responses
  - Maintenance of cone function over time
  - Preservation of retinal structure, including outer retinal thickness and photoreceptor morphology
- Identified minimal effective dose and evidence of durability across timepoints
- Data also suggest interocular vector transfer, indicating potential systemic distribution dynamics

These results support a differentiated strategy to treat genetically heterogeneous retinal diseases with a single therapeutic approach.

The presentations and posters will be available on the Opus Genetics website here.

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 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 Phentolamine Ophthalmic Solution 0.75%, OPGx-LCA5, OPGx-BEST1, RDH12,

MERTK, RHO, CNGB1 and NMNAT1, and earlier stage programs, 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, our subsequent Quarterly Reports on Form 10-Q, 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.

Contacts:
Investors
Jenny Kobin
Remy Bernarda
IR Advisory Solutions
ir@opusgtx.com

Media
Kimberly Ha
KKH Advisors
917-291-5744
kimberly.ha@kkhadvisors.com

Source: Opus Genetics, Inc.

Source: Opus Genetics, Inc.