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# Opus Genetics Highlights 2025 Progress and Upcoming 2026 Catalysts

- Multi-asset pipeline with significant targeted data readouts and milestones -
- Two lead ophthalmic gene therapy programs in clinical trials with new data anticipated in 2026 -
- Supplemental New Drug Application (sNDA) submitted for partnered presbyopia treatment -
- Corporate presentation at J.P. Morgan Healthcare Conference on Thursday, January 15, at 8:15 a.m. PT -

RESEARCH TRIANGLE PARK, N.C., Jan. 08, 2026 (GLOBE NEWSWIRE) -- [Opus Genetics, Inc.](#) (Nasdaq: IRD) (the "Company," "Opus," or "Opus Genetics"), a clinical-stage biopharmaceutical company developing gene therapies to restore vision and prevent blindness in patients with inherited retinal diseases (IRDs), today highlighted its progress achieved during 2025 and upcoming catalysts in 2026.

In 2026, Opus is expected to announce clinical data from its BEST1 program and accelerate its LCA5 program into pivotal testing and toward potential U.S. Food and Drug Administration (FDA) approval. Additional programs are also expected to advance into the clinic. Most of Opus' pipeline programs have the potential to qualify for Rare Pediatric Disease designation, which could result in Priority Review Vouchers (PRV), providing future non-dilutive capital.

"2025 was a year defined by strong execution at Opus," said George Magrath, M.D., Chief Executive Officer, Opus Genetics. "We achieved aggressive milestones across multiple ophthalmic programs – advancing two gene therapies in clinical trials and submitting an application to the FDA for approval of our second partnered commercial product, which could potentially provide a large new market opportunity. We secured capital from leading institutional investors and through non-dilutive sources such as patient advocacy grant funding. We believe that the progress we've made with our LCA5 and BEST1 programs is proof of what's possible and our broader pipeline is poised to follow. We enter 2026 with confidence, momentum, and an unwavering commitment to patients who inspire everything we do."

## Corporate Updates

### OPGx-BEST1 – Gene Therapy for BEST1-Related IRD

- Potential treatment for both the dominant and recessive forms of BEST1 disease.

- First participant dosed in Phase 1/2 trial (BIRD-1) with enrollment ongoing.
- Initial data expected this quarter at Macula Society with 3-month results from the entire Cohort 1 expected in mid-2026.
- OPGx-BEST1 is potentially eligible for multiple regulatory designations which the Company expects to file for in 2026.

### **OPGx-LCA5 – Gene Therapy for Leber Congenital Amaurosis (LCA)**

- Multiple regulatory designations granted including Rare Pediatric Disease, Orphan Drug, and Regenerative Medicine Advanced Therapy (RMAT) with potential eligibility for Priority Review Voucher upon approval.
- Positive Phase 1/2 safety and efficacy data reported in adults and pediatric participants.
- Enrollment ongoing in run-in period for planned, adaptive pivotal Phase 3 trial.
- Dosing with OPGx-LCA5 in the Phase 3 trial expected in the second half of 2026.

### **Pre-Clinical Gene Therapy Pipeline**

- Multiple partnerships with patient advocacy organizations are increasing awareness in the IRD community and providing non-dilutive funding for pre-clinical testing of Opus Genetics gene therapy pipeline.
- Promising programs for IRDs related to genetic mutations in RHO, CNGB1, MERTK, RDH12-LCA, and NMNAT1 with one to two programs targeted to enter clinical testing this year.

### **Phentolamine Ophthalmic Solution 0.75% (PS)**

- Supplemental New Drug Application (sNDA) submitted for the treatment of presbyopia, with an anticipated regulatory decision by the end of 2026.
- LYNX-3, the second pivotal Phase 3 trial in keratorefractive participants with visual disturbances under mesopic, low-contrast conditions, is ongoing with topline results expected in the first half of 2026.

### **Financial Outlook**

As of September 30, 2025, Opus Genetics had cash and cash equivalents of \$30.8 million. Subsequent to the end of the third quarter of 2025, the Company raised approximately \$23.0 million in gross proceeds through a registered direct offering of equity securities, resulting in a total cash position of over \$50 million. Based on current operating plans, the Company expects its existing cash resources will fund operations into the second half of 2027, excluding any potential proceeds from callable warrants or future milestone payments.

### **About OPGx-BEST1 and the Phase 1/2 Trial**

OPGx-BEST1 leverages Opus Genetics' proprietary AAV-based gene therapy platform, designed to deliver a functional copy of the BEST1 gene directly to the retinal pigment epithelium (RPE) cells where the defective gene resides. The program builds on extensive preclinical work demonstrating restoration of BEST1 protein expression and improved retinal function in relevant disease models. The multi-center, adaptive, open-label, dose-exploring Phase 1/2 trial, known as BIRD-1, will evaluate the safety, tolerability, and preliminary

efficacy of OPGx-BEST1 in participants with Best Vitelliform Macular Dystrophy (BVMD) or Autosomal-Recessive Bestrophinopathy (ARB). Treatment will be administered via a single subretinal injection in one eye of each participant with two dosing cohorts. The trial will also explore biological activity through functional and anatomical endpoints, including changes in visual function and retinal structure.

### **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 advanced into a pivotal Phase 3 trial. 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 received Rare Pediatric Disease, Orphan Drug and 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 RHO, CNGB1, RDH12, NMNAT1, and MERTK. Opus Genetics is also advancing Phentolamine Ophthalmic Solution 0.75%, an approved small-molecule therapy for pharmacologically induced mydriasis, with additional potential indications in presbyopia and low-light visual disturbances following keratorefractive surgery. The Company is based in Research Triangle Park, NC. For more information, visit [www.opusgtx.com](http://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 cash runway, the clinical development, clinical results, preclinical data, and future plans for Phentolamine Ophthalmic Solution 0.75%, OPGx-LCA5, OPGx-BEST1, RDH12, 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, 2024, our subsequent Quarterly Report on Form 10-Q, and 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,” “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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