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Opus Genetics Announces Dosing of First Participant in OPGx-BEST1 Phase 1/2 Gene Therapy Clinical Trial for Best Disease

Emerging gene therapy administered through a one-time subretinal injection marks a major milestone for the inherited retinal disease community

Initial data expected in Q1 2026

RESEARCH TRIANGLE PARK, N.C., Nov. 13, 2025 (GLOBE NEWSWIRE) -- Opus Genetics, (Nasdaq: IRD) (the "Company" 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 announced that the first participant has been dosed in the Company's OPGx-BEST1 Phase 1/2 clinical trial for Best disease (BEST1).

Best disease, or vitelliform macular dystrophy, is a rare, inherited retinal condition causing macular degeneration by mutations in the BEST1 gene, leading to progressive vision loss and, in some cases, blindness. OPGx-BEST1 is an emerging gene therapy administered through a one-time subretinal injection designed to restore function to the retinal pigment epithelium (RPE) cells affected by mutations in the BEST1 gene.

The Phase 1/2 trial is being conducted by Dr. Mark Pennesi of the Retina Foundation of the Southwest and the surgical team of Drs. Kenneth Fan and Charles Wykoff at Retina Consultants of Texas.

"Dosing the first participant in our OPGx-BEST1 program is a historic moment for the BEST disease community and for our team at Opus," said George Magrath, M.D., Chief Executive Officer, Opus Genetics. "This milestone reinforces our mission to develop one-time gene therapies for inherited retinal diseases that previously had no treatment options. It's a privilege to collaborate with leaders in the field like Drs. Pennesi, Fan, and Wykoff. We are deeply thankful to the Retina Foundation of the Southwest, Retina Consultants of Texas, and most importantly, the patients and families who place their trust in us. Together, we are striving to build a future where no one loses their sight to inherited retinal disease."

"This milestone highlights the significant progress being made in ophthalmic gene therapy and the potential power of collaboration between industry and academia to bring new hope to families affected by inherited retinal diseases," said Dr. Mark Pennesi, Director of the Inherited Retinal Degeneration Clinic at the Retina Foundation of the Southwest. "For

patients with Best disease, this represents an important step toward potentially preserving and restoring vision."

"The successful dosing of the first patient underscores both the potential promise of gene therapy in ophthalmology and the dedication of the entire community working to make these treatments a reality," said Dr. Charles Wykoff, Surgical Retina Specialist and Ophthalmologist at the Retina Consultants of Texas. "It's inspiring to see years of scientific progress translate into potentially meaningful advances for patients and families."

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 study, 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. Initial data from the trial is expected in the first quarter of 2026.

About BEST1 Inherited Retinal Disease

The BEST1 gene is responsible for providing instructions to produce bestrophin, a protein that acts as a channel to manage the movement of charged chloride ions in and out of retinal cells. Variants (mutations) in the BEST1 gene, as well as the PRPH2 gene, can result in the formation of abnormally shaped channels that cannot properly control chloride flow. BEST1 plays a key role in the retinal pigment epithelium (RPE), which is essential for healthy vision, and such mutations can lead to BEST1-related inherited retinal diseases (bestrophinopathies). These rare conditions affect an estimated 9,000 patients across the United States and can lead to progressive vision loss and blindness.

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, RDH12, and MERTK. Opus Genetics is also advancing Phentolamine Ophthalmic Solution 0.75%, an approved small-molecule therapy for pharmacologically induced mydriasis, with additional indications in late-stage development for 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.

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 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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