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Opus Genetics Announces Initial Clinical Data from Phase 1/2 OPGx-BEST1 Gene Therapy Study at the Macula Society Annual Meeting

- *Sentinel participant showed OPGx-BEST1 was well tolerated with no ocular inflammation, treatment-related adverse events, or dose-limiting toxicities at three months*

- *Early signals of functional and structural improvement observed at one month and three months*

- *12-letter BCVA gain and 23% CST reduction observed in the treated eye at three months*

- *Full cohort data expected in mid-year 2026*

RESEARCH TRIANGLE PARK, N.C., Feb. 27, 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), announced today new clinical data from its ongoing Phase 1/2 study of OPGx-BEST1 gene therapy, presented at the 49th Annual Meeting of the Macula Society, in San Diego, California.

The presentation, titled "Preliminary Results from an Adult Participant in a Phase 1b/2a Clinical Study of OPGx-BEST1 Gene Therapy for the Treatment of BVMD and ARB Due to BEST1 Mutations," reported 3-month results from the first (sentinel) adult participant treated in the study, highlighting positive safety, tolerability, and biological activity following subretinal administration of OPGx-BEST1.

The sentinel participant is a 63-year-old female with Autosomal-Recessive Bestrophinopathy (ARB) disease with severe functional impairment. The data demonstrated that OPGx-BEST1 was well tolerated with no ocular inflammation, no ocular or treatment-related adverse events, and no dose limiting toxicities. Early signals of functional vision improvement were observed, including an equivalent 12-letter gain in Best Corrected Visual Acuity (BCVA) in the treated study eye. In addition, structural improvement in central subfield thickness (CST) was observed with a 23% decrease in the study eye. Resolution of intraretinal fluid was also

seen as early as 1-month in areas with less atrophy.

“We are encouraged by these results from our sentinel participant, showing OPGx-BEST1 was well-tolerated and demonstrated promising initial efficacy at three months,” said George Magrath, M.D., Chief Executive Officer, Opus Genetics. Although early, this data represents an important milestone for our OPGx-BEST1 program and for patients with BEST1-related retinal diseases.”

“BEST1-related retinal diseases represent a significant unmet medical need, with no approved treatments currently available,” said Mark Pennesi, M.D., Ph.D., study investigator at the Retina Foundation of the Southwest in Dallas, Texas. “The preliminary results from this study, including the early favorable safety profile and initial signals of functional and structural improvement, are encouraging and support continued evaluation of OPGx-BEST1 as a gene augmentation approach for patients with BEST1-associated disease.”

Recruitment in the Phase 1/2 study is ongoing at two clinical sites in the U.S., with additional sites expected to open in Florida, Cincinnati and New York. Two participants have been enrolled to date, with 3-month results from the full Cohort 1 expected in mid-year 2026.

The full presentation and video recording will be available on the Opus Genetics website in the Events section.

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.

By restoring BEST1 function, the therapy aims to address the underlying genetic cause of retinal degeneration and support preservation of photoreceptor health and visual function. BEST1-associated IRDs affect an estimated 22,000 patients worldwide and currently have no approved treatments.

The ongoing adaptive, open-label Phase 1/2 study is evaluating single-eye subretinal administration of OPGx-BEST1 up to two dose levels in adult 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 primary objective is to assess safety and tolerability and identify the most appropriate dose for further clinical development, with participants followed longitudinally for long-term outcomes. The trial will also explore biological activity through functional and anatomical endpoints, including changes in visual function and retinal structure.

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.

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, 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 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.

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