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# Opus Genetics Announces Positive Recommendation from Independent Data Monitoring Committee for Phase 1/2 Trial in Best Disease

## Initial favorable safety profile demonstrated in OPGx-BEST1 clinical trial

RESEARCH TRIANGLE PARK, N.C., Dec. 09, 2025 (GLOBE NEWSWIRE) -- Opus Genetics (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 the Independent Data Monitoring Committee (IDMC) issued a positive recommendation to continue as planned in the Company's Phase 1/2 BEST1 clinical trial (BIRD-1), which is a multi-center, adaptive, open-label, dose-exploring study evaluating OPGx-BEST1 in participants with Best disease.

The IDMC overseeing the trial completed its pre-specified safety review of the one-month data from the sentinel participant and recommended advancing enrollment and dosing of additional participants in the trial, without modification.

"We are thrilled with this outcome from the first participant, whose encouraging safety results at one month enable us to proceed with dosing the next four participants in our BEST1 Phase 1/2 trial," said George Magrath, M.D., Chief Executive Officer, Opus Genetics. "The IDMC's safety review and recommendation to continue the trial reinforces our confidence as we advance this program. BEST1-associated retinal diseases constitute a substantial unmet medical need, and this progress with OPGx-BEST1 represents an important step toward potentially preserving and restoring visual function for patients with Best disease."

**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.

**About BEST1 Inherited Retinal Disease**

Best disease, or vitelliform macular dystrophy, is a rare inherited retinal condition caused by mutations in the BEST1 gene, leading to impaired retinal pigment epithelium (RPE) function, progressive vision loss, and, in some cases, blindness. 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 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, please 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 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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