July 23, 2025

Opus Genetics and the Global RDH12 Alliance Partner to Advance RDH12 Gene Therapy for Inherited Childhood Blindness

- *Collaboration aims to accelerate development of OPGx-RDH12, a gene therapy for RDH12-associated Leber congenital amaurosis (RDH12-LCA)*

RESEARCH TRIANGLE PARK, N.C., July 23, 2025 (GLOBE NEWSWIRE) -- Opus Genetics, Inc. (Nasdaq: IRD), a clinical-stage biopharmaceutical company developing gene therapies for the treatment of inherited retinal diseases (IRDs) and small molecule therapies for other ophthalmic disorders, today announced a strategic partnership with the Global RDH12 Alliance (the Alliance) to advance Opus' gene therapy program for patients with vision loss due to retinol dehydrogenase 12 (RDH12) gene mutations. The Alliance serves as a collaborative platform uniting key advocacy groups dedicated to RDH12-related IRDs, including: (i) "RDH12 Fund for Sight" in the U.S. and (ii) "Eyes on the Future" in the UK.

This collaboration will accelerate development of OPGx-RDH12, Opus' gene therapy program targeting the RDH12 gene mutation for the potential treatment of Leber congenital amaurosis (RDH12-LCA). Leber congenital amaurosis (LCA) is a rare IRD that causes progressive vision loss and blindness, often beginning in early childhood. Patients with RDH12 mutations often have early visual acuity loss with retinal structural changes by two years of age.

Under the agreement, the Alliance will provide up to $1.6 million towards the development of the OPGx-RDH12 program. The partnership also includes a risk-sharing structure and performance-based milestones. Together, the parties will co-develop the OPGx-RDH12 program, including the clinical and regulatory strategy, with the goal of filing an Investigational New Drug (IND) application with the U.S. Food and Drug Administration (FDA) by late 2025.

"Since founding the RDH12 Fund for Sight more than a decade ago, our goal has always been to bring a treatment to the RDH12-LCA community," said Mathew Pletcher, Ph.D., Board member of the RDH12 Fund for Sight and father to a 19-year-old living with the condition. "This partnership represents a significant step forward. By combining our patient community's unique, first-hand perspectives on RDH12-LCA and resources with Opus' gene therapy expertise, we can accelerate the transition of this promising therapy out of the laboratory and into the clinic."

Silvia Cerolini, CEO of Eyes on the Future and mother of an 11-year-old girl affected by RDH12-LCA, added, "We are racing against time as our children's vision continues to deteriorate. Partnering with Opus to bring this gene therapy into the clinic is an incredibly

meaningful milestone, and it shows what's possible when patients and the industry work together. It brings us one step closer to our mission: helping our kids and our community see the world for longer."

"Opus is pleased to work directly with the patient community in true partnership," said George Magrath, CEO of Opus Genetics. "This collaboration is much more than a financial arrangement. We value each other's insights, experience and connections as critical to a successful co-development of this gene therapy."

Professor Jean Bennett, MD, PhD, Opus Genetics Scientific Advisor and Board of Directors member, added, "With the understanding of how gene therapy works and the preclinical evidence so far, we see a clear path for its application to RDH12-LCA. I want to thank everybody in the RDH12 community for their indispensable support to RDH12 research over the years. This partnership is a critical enabler to accelerate the path to bringing this therapy to all of those in need."

About RDH12-LCA and OPGx-RDH12

RDH12-LCA is an ultra-rare form of childhood blindness affecting several thousand people globally. Mutations in the RDH12 gene impair protein function in the retina, leading to early visual decline, often with structural retinal changes by age two, and rapid progression during the second decade of life. OPGx-RDH12 uses an adeno-associated virus (AAV) vector to deliver a functional copy of the RDH12 gene directly to photoreceptors in the retina. "Preclinical studies in cell and mouse models have shown restoration of RDH12 activity and functional improvements," said Professor Jean Bennett, MD, PhD, Opus Genetics Scientific Advisor and Board of Directors member.

About RDH12 Fund for Sight

The RDH12 Fund for Sight is a U.S.-based not-for-profit organization that aims to bring together families with common concerns and worries, collect and distribute information about the form of LCA caused by RDH12, and, most importantly, ensure that significant funds are devoted to the study and development of treatments for LCA caused by RDH12. More information is available at http://rdh12.org.

About Eyes on the Future

Eyes on the Future is a UK-based non-for-profit organization supporting research into rare diseases and eye diseases with specific focus on RDH12 inherited retinal dystrophies. Funded by Enrico and Silvia, whose 11-year-old child is affected by RDH1- LCA, Eyes on the Future is a registered Company limited by guarantee in England and Wales, No. 13956181 and a registered Charity No. 1198330. More information is available at www.eyesonthefuture.org.uk.

About Opus Genetics

Opus Genetics is a clinical-stage biopharmaceutical company developing gene and small molecule therapies for vision-threatening eye diseases. Opus' pipeline features AAV-based gene therapies targeting inherited retinal diseases including LCA, bestrophinopathy, and retinitis pigmentosa. Its lead candidate, OPGx-LCA5, is in a Phase 1/2 trial for LCA5-related

mutations and has shown encouraging early results. Additional programs include OPGx-BEST1, a gene therapy targeting BEST1-related retinal degeneration and a Phase 3-ready small molecule therapy for diabetic retinopathy, developed under a Special Protocol Assessment with the FDA. Opus is also advancing Phentolamine Ophthalmic Solution 0.75%, a partnered therapy currently approved in one indication and is being studied in two Phase 3 programs for presbyopia and dim light vision disturbances. Opus 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, 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 Quarterly Report on Form 10-Q for the quarter ended March 31, 2025, 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 hereof. 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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