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Opus Genetics Announces Agreement with Massachusetts Eye and Ear and Harvard Medical School to License Third Program for Inherited Retinal Disease

Program targets NMNAT1 gene based on the work of Opus scientific founder Dr. Eric Pierce

RALEIGH, N.C., Nov. 11, 2021 (GLOBE NEWSWIRE) -- Opus Genetics, a patient-focused gene therapy company developing treatments for orphan inherited retinal diseases, today announced an agreement to license its third preclinical program to address mutations in the NMNAT1 gene, which cause a specific form of Leber congenital amaurosis (LCA), from Massachusetts Eye and Ear, the primary teaching hospital at Harvard Ophthalmology.

The new program, OPGx-003, is based on the work of Eric Pierce, M.D., Ph.D., Director of the Ocular Genomics Institute and William F. Chatlos Professor of Ophthalmology at Massachusetts Eye and Ear and Harvard Medical School, and scientific co-founder of Opus. OPGx-003 is a gene augmentation therapy designed to halt functional deterioration in pediatric patients with retinal degenerative disease caused by mutations in the nicotinamide mononucleotide adenylyltransferase 1 (NMNAT1) gene. Recent preclinical data have demonstrated the potential for this gene augmentation approach to achieve stable rescue of retinal structure and function. Opus expects to file an IND for OPGx-003 in the first half of 2023.

“We’re thrilled to collaborate with Dr. Pierce, who will bring his expertise in retinal gene therapy to researching a novel treatment of NMNAT1-associated retinal degeneration,” said Ash Jayagopal, Ph.D., Chief Scientific Officer of Opus Genetics. “Adding OPGx-003 to the Opus pipeline further underscores our commitment to bringing the required resources and expertise together to take promising science from the lab through the clinic and ultimately to patients who need it, and reinforces the importance of our innovative patient-focused model.”

“I’ve seen firsthand the need for new treatments for rare inherited retinal diseases,” said Dr. Pierce. “As someone who has devoted my life to research in this space, I believe strongly that Opus is an ideal company to advance this work to make a difference for patients in need.”

Opus’s lead program, OPGx-001, is designed to address mutations in the LCA5 gene, which encodes the lebercilin protein. The company’s second program, OPGx-002, will focus on restoring protein expression and halting functional deterioration in patients with retinal

dystrophy caused by mutations in the retinal dehydrogenase (RDH12) gene (LCA13).

About Opus Genetics
Opus Genetics is a groundbreaking gene therapy company for inherited retinal diseases with a unique model and purpose. Backed by Foundation Fighting Blindness's venture arm, the RD Fund, Opus combines unparalleled insight and commitment to patient need with wholly owned programs in numerous orphan retinal diseases. Its AAV-based gene therapy portfolio tackles some of the most neglected forms of inherited blindness while creating novel orphan manufacturing scale and efficiencies. Based in Raleigh, N.C., the company leverages knowledge of the best science and the expertise of pioneers in ocular gene therapy to transparently drive transformative treatments to patients. For more information, visit www.opusgenetics.com.

Disclosures
Dr. Pierce holds equity in and serves as a consultant for Opus Genetics.

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