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Opus Genetics Announces Acquisition of the Rights to Two Gene Therapy Product Candidates for Inherited Retinal Diseases

Opus to advance preclinical development programs for BEST1- *and* RHO-*related retinal diseases*

Deal expands Opus' addressable patient population for its novel treatments for rare inherited retinal diseases

RESEARCH TRIANGLE PARK, N.C., Dec. 28, 2022 (GLOBE NEWSWIRE) -- Opus Genetics, a patient-first, clinical-stage gene therapy company developing treatments for inherited retinal diseases, today announced it has acquired the rights to two preclinical-stage AAV-based gene therapy product candidates for inherited retinal diseases (IRDs) from Iveric Bio. Opus will develop the novel gene therapy candidates to address *bestrophin-1* (*BEST1*)-related inherited retinal diseases and rhodopsin-mediated autosomal dominant retinitis pigmentosa (*RHO*-adRP), respectively.

BEST1-related IRDs are estimated to affect approximately one in 69,000 people, or nearly 5,000 people, in the United States. The *BEST1* gene therapy is designed to deliver a functional copy of the *BEST1* gene to retinal pigment epithelial cells to produce bestrophin-1 protein and normalize homeostasis between the photoreceptors and retinal pigment epithelial cells. *RHO*-adRP is one of the most common IRDs, estimated to affect approximately one in 51,000 people, or more than 6,000 people, in the United States alone.

"The addition of these innovative *BEST1* and *RHO*-adRP programs significantly increases the patient population that could benefit from Opus therapies and complements our existing pipeline of gene therapies for inherited retinal diseases," said Ben Yerxa, PhD, Chief Executive Officer of Opus. "Opus is building an engine for addressing multiple IRDs, and deals like this one help us achieve our desire for sustainable growth of our infrastructure, operations, scientific expertise and ultimately our patient impact, with the goal of at least one IND per year."

Opus anticipates completing additional IND-enabling studies and filing an IND for *BEST1* in the second half of 2023.

As part of the deal, Opus will assume responsibility for the global research, development and commercialization of *BEST1* and *RHO*-adRP programs. In exchange, Iveric received an upfront payment of $500,000 and high single-digit percentage ownership of Opus. Iveric is also eligible to receive development and regulatory milestone payments, sales milestone payments, and a low single-digit earnout on net sales of the products. Iveric retains certain

rights with respect to the potential future commercialization of gene therapy products for *BEST1* and/or *RHO*-adRP under certain circumstances.

“Affecting over 28,000 people across the U.S., EU and UK, *BEST1* and *RHO*-adRP represent a significant portion of all inherited retinal diseases and an urgent unmet need for effective treatment. IRDs are ideal targets for genetic therapies to stop the retinal degeneration and improve the lives of patients living with severe vision loss or blindness,” said Bart P Leroy, MD, PhD, head of the Department of Ophthalmology, professor of Ophthalmology and member of the Center for Medical Genetics at Ghent University and Ghent University Hospital in Belgium; and attending physician in the Division of Ophthalmology and The Raymond G. Perelman Center for Cellular and Molecular Therapeutics at the Children’s Hospital of Philadelphia.

About Opus Genetics
Opus Genetics is a clinical-stage gene therapy company for inherited retinal diseases with a unique model and purpose. Backed by Foundation Fighting Blindness’ 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 Research Triangle Park, 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.

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