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Opus Genetics Announces Promising New Data Highlighting Potential of AAV-based Gene Therapies for the Treatment of Rare Inherited Retinal Diseases

Retinal regions of preserved photoreceptors identified as targets for subretinal delivery of AAV8-based gene therapy to address mutations in genes that cause forms of Leber congenital amaurosis

Subretinal injection well tolerated in preclinical dose-ranging studies

Data presented at Association for Research in Vision and Ophthalmology (ARVO) Annual Meeting 2022

RESEARCH TRIANGLE PARK, N.C., May 04, 2022 (GLOBE NEWSWIRE) -- Opus Genetics, a patient-focused gene therapy company developing treatments for inherited retinal diseases, today announced promising new preclinical data from studies evaluating the potential of its gene therapies OPGx-001 and OPGx-002 to address forms of Leber congenital amaurosis (LCA), a group of rare inherited retinal diseases characterized by photoreceptor degeneration, due to mutations of *LCA5* or *RDH12* genes, respectively.

In preparation for IND-enabling trials of OPGx-001 and OPGx-002, studies were conducted to determine eligibility, therapeutic window, and possible outcome measures for gene therapy for *LCA5* and *RDH12* inherited retinal diseases. In addition, safety evaluations for the subretinal delivery of an AAV8 vector containing *LCA5* or *RDH12* were performed in non-human primates (NHP).

The data demonstrated that despite severe retinal dysfunction, LCA patients exhibited detectable photoreceptor regions that may be targets for gene augmentation, identified in the central and midperipheral retina of *LCA5*-LCA patients and in the pericentral and peripapillary retina of *RDH12*-LCA patients. In two dose-ranging studies in NHPs, subretinal delivery of OPGx-001 and OPGx-002 was well tolerated, with mild inflammatory changes observed at the higher dose. The data support the therapeutic potential and tolerability of gene augmentation to address *LCA5*-LCA and *RDH12*-LCA and provide guidance for formal preclinical toxicology studies and future human clinical trials.

“Patients with Leber congenital amaurosis due to mutations of the*LCA5* or *RDH12* genes experience rapid retinal degeneration, resulting in vision loss in early childhood,” said Ash Jayagopal, Ph.D., Chief Scientific Officer of Opus. “The detection of preserved photoreceptors in LCA patients signals a therapeutic opportunity to target the mutation and

potentially restore structure and function through gene augmentation. In addition, the encouraging dose-ranging results in the primate model suggest subretinal delivery of Opus' AAV8-based gene therapies are safe and inform the doses to be used in our toxicology studies, a key step on our path toward the clinic for OPGx-001 and OPGx-002."

The data were presented today at the Association for Research in Vision and Ophthalmology (ARVO) Annual Meeting 2022 in Denver, Colo., by Jean Bennett, M.D., Ph.D., Scientific Co-founder, Opus Genetics, and University of Pennsylvania Perelman School of Medicine; and Tomas Aleman, M.D., University of Pennsylvania Perelman School of Medicine.

Also at ARVO 2022, Dr. Jayagopal was awarded the title of ARVO Fellow, an honor established to recognize current ARVO members for their individual accomplishments, leadership and contributions to the ARVO Association.

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 retinal diseases. Its AAV-based gene therapy portfolio tackles some of the most neglected forms of inherited blindness while creating novel 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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