

November 3, 2023



New Preliminary Clinical Data on Potential of Opus AAV-based Gene Therapy for Rare Inherited Retinal Disease to be Presented at the American Academy of Ophthalmology Annual Conference 2023

Data to be presented as part of gene augmentation therapy presentation

RALEIGH, N.C., Nov. 03, 2023 (GLOBE NEWSWIRE) -- Opus Genetics, a patient-focused gene therapy company developing treatments for inherited retinal diseases, today announced that preliminary data from a Phase 1/2 clinical trial evaluating the potential of its gene therapy to address Leber congenital amaurosis (LCA) due to mutations in *LCA5* will be presented in a symposium at the American Academy of Ophthalmology annual conference held November 3-6 in San Francisco. The data will be presented as part of a broader presentation by Tomas S. Aleman, MD, the principal investigator of the trial, focused on gene augmentation therapies for the treatment of inherited retinal degenerations.

Details of the presentation are as follows:

Title: Gene Augmentation Therapy for Inherited Retinal Diseases

Session: The Future of Retinal Disease Pharmacological, Stem Cell and Gene Therapy Treatments

Location: In-Person Session SYM47, Moscone Center WEST3018, Live Broadcast, On Demand

Date / time: Sunday, November 5, 2023, 11:30 a.m. – 12:45 p.m. PST

Presenter: Dr. Aleman, Department of Ophthalmology, University of Pennsylvania Perelman School of Medicine

About OPGx-LCA5

OPGx-LCA5 is designed to address a form of Leber congenital amaurosis (LCA) due to biallelic mutations in the *LCA5* gene (*LCA5*), which encodes the lebercilin protein. *LCA5* is an early-onset severe inherited retinal dystrophy. Studies in *LCA5* patients have reported evidence for the dissociation of retinal architecture and visual function in this disease, suggesting an opportunity for therapeutic intervention through gene augmentation. OPGx-LCA5 uses an adeno-associated virus 8 (AAV8) vector to precisely deliver a functional *LCA5* gene to the outer retina. Preclinical data, including animal and human iPSC models, have

demonstrated preservation of retinal structure and visual function when OPGx-LCA5 was administered prior to peak disease severity.

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.

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Source: Opus Genetics