Positive clinical trial results on gene transfer to treat macular degeneration

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The long-term safety of gene transfer to treat neovascular age-related macular degeneration (NVAMD), and the production of two therapeutic proteins encoded by those genes for at least 2.5 years in the eyes of patients with advanced NVAMD are demonstrated in the results of a clinical trial published in *Human Gene Therapy*.

In the article entitled "Lentiviral Vector Gene Transfer of Endostatin/Angiostatin for Macu-lar Degeneration (GEM) Study," Peter Campochiaro, MD and coauthors from The Wilmer Eye Institute, Johns

Hopkins University School of Medi-cine (Baltimore, MD), Casey Eye Institute, Oregon Health & Science University (Portland, OR), University of Iowa Hospitals and Clinics (Iowa City), and Oxford BioMedica (Oxford, U.K.) present the design and results of the Phase 1 clinical trial. Each study participant re-ceived treatment in one eye consisting of a subretinal injection of a viral vector engineered to deliver two therapeutic genes, endostatin and angiostatin, each of which blocks the for-mation of blood vessels to combat the progression of NVAMD.

"It has been the hope that the spectacular success of gene therapy for genetic retinal disor-ders would translate into a platform for treating more common diseases of the eye," says Editor-in-Chief Terence R. Flotte, MD, Celia and Isaac Haidak Professor of Medical Edu-cation and Dean, Provost, and Executive Deputy Chancellor, University of Massachusetts Medical School, Worcester, MA. "Dr. Campochiaro's pioneering work on age-related macu-lar degeneration (AMD) demonstrates the feasibility of such an approach for one of the most common vision-threatening disorders, and one whose incidence is rising dramatically as our population ages."

More information: Peter A. Campochiaro et al, Lentiviral Vector Gene Transfer of Endostatin/Angiostatin for Macular Degeneration (GEM) Study, *Human Gene Therapy* (2016). DOI: 10.1089/hum.2016.117

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