Gene therapy fully restores vision in mouse model of Leber congenital amaurosis

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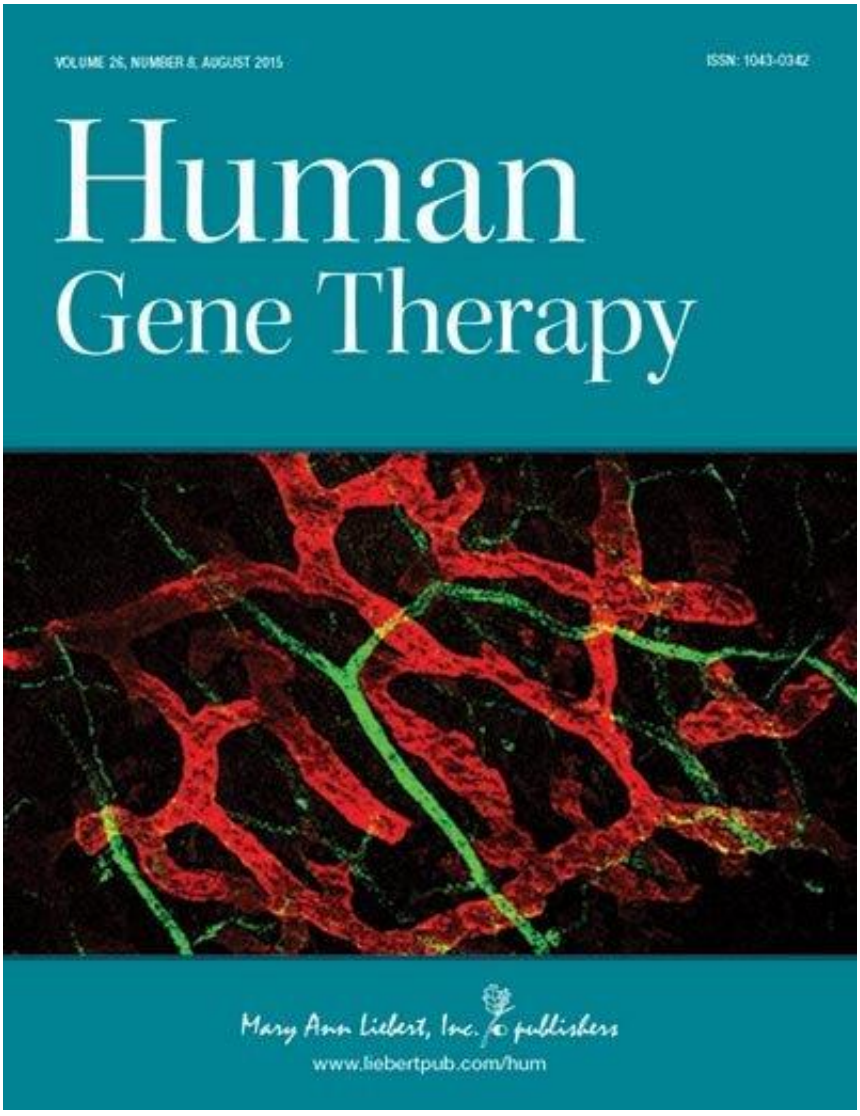

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Mice lacking the protein retGC1, which is deficient in humans suffering Leber congenital amaurosis-1 (LCA1), a disorder that causes severe visual impairment beginning in infancy, received gene therapy to replace retGC1 and showed fully restored visual function that persisted for at least 6 months. The success of this approach strongly support clinical testing of a gene therapy targeted to the retinas of LCA1 patients, conclude the authors of the study published in *Human Gene Therapy*.

Sanford Boye, Shannon Boye, and coauthors from University of Florida College of Medicine, Gainesville, University of Oklahoma College of Medicine, Oklahoma City, and Salus University, Elkins Park, PA, emphasize the need for a treatment strategy targeting the loss of cone function that occurs in the eyes of patients with LCA1. They describe a gene replacement approach that uses an adeno-associated viral (AAV) vector to deliver the gene encoding the retGC1 protein to the cone-rich central retina in an all-cone mouse model deficient in retGC1. They report the study design, results, and their conclusions in the article "Gene Therapy Fully Restores Vision to the All-Cone Nrl–/–Gucy2e–/– Mouse Model of Leber Congenital Amaurosis-1."

"This study shows the tremendous potential of recombinant (rAAV) gene therapy for the effective treatment of genetic causes of vision loss," says Editor-in-Chief Terence R. Flotte, MD, Celia and Isaac Haidak Professor of Medical Education and Dean, Provost, and Executive Deputy Chancellor, University of Massachusetts Medical School, Worcester, MA.

More information: The article is available free on the *Human Gene Therapy* website until September 30, 2015.

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