Mini form of replacement gene can delay degeneration in leber congenital amaurosis

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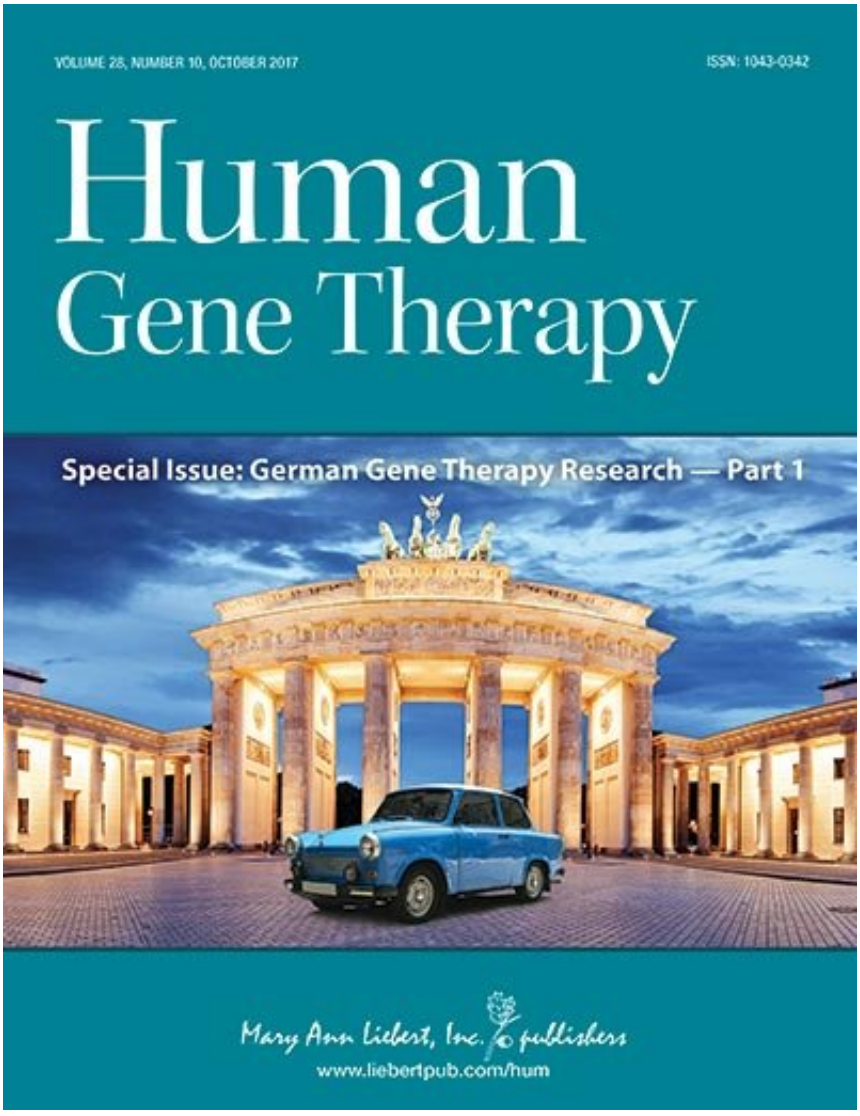

Credit: Mary Ann Liebert, Inc., publishers

A new study demonstrates success in using a shortened form of the CEP290 gene for gene therapy in a mouse model of Leber congenital amaurosis type 10 (LCA10), a retinal degenerative disorder that causes childhood blindness. The large size of the full-length gene has proven difficult to package into the adeno-associated virus (AAV) delivery vectors commonly used in gene therapy, but use of the truncated gene led to significant improvement in photoreceptor survival, morphology, and function, as reported in an article published in *Human Gene Therapy*.

Wei Zhang, Linjing Li, Qin Su, Guangping Gao, and Hemant Khanna, University of Massachusetts Medical School, Worcester, describe their work in the article entitled "Gene Therapy Using a miniCEP290 Fragment Delays Photoreceptor Degeneration in a Mouse Model of Leber Congenital Amaurosis." The researchers present not only a potential approach for designing a suitable gene therapeutic strategy to overcome the CEP290 gene mutation underlying LCA, but also highlight the use of therapeutic minigenes as a viable option for delivering large genes to treat other diseases.

"The dramatic clinical results observed in patients treated with AAV-RPE65 vectors with LCA2 has raised expectations that similar approaches could be used to treat many different single gene disorders of the retina," 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. "Overcoming the limitation of the packaging capacity of the AAV particle for this large gene is a critical step in the development of LCA10 gene therapy."

More information: Wei Zhang et al, Gene Therapy Using a miniCEP290 Fragment Delays Photoreceptor Degeneration in a Mouse Model of Leber Congenital Amaurosis, *Human Gene Therapy* (2017).

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