New gene therapy strategies emerging to combat vision loss

22 June 2016

Diseases of the eye that cause vision loss and blindness, especially neurodegenerative disorders affecting the retina, are ideal targets for gene therapy, including gene replacement and promising corrective gene editing strategies. A comprehensive Review article providing an overview of emerging therapeutic approaches and innovative gene delivery and gene editing tools to treat ocular diseases is published in *Human Gene Therapy*.

Lolita Petit, Hemant Khanna, and Claudio Punzo, University of Massachusetts Medical School, Worcester, coauthored the article "Advances in Gene Therapy for Diseases of the Eye." They provide insights on recent progress in clinical applications of retinal gene replacement therapy, preclinical advances in gene-specific therapy for photoreceptor diseases, and the development of gene independent therapeutic strategies.

"The spectacular successes of gene therapy for eye diseases may be seen as providing a platform for molecular approaches to a much broader range of disorders affecting vision," 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: Lolita Petit et al, Advances in Gene Therapy for Diseases of the Eye, *Human Gene Therapy* (2016). DOI: 10.1089/hum.2016.040