Clinical advances in gene therapy for central nervous system disorders

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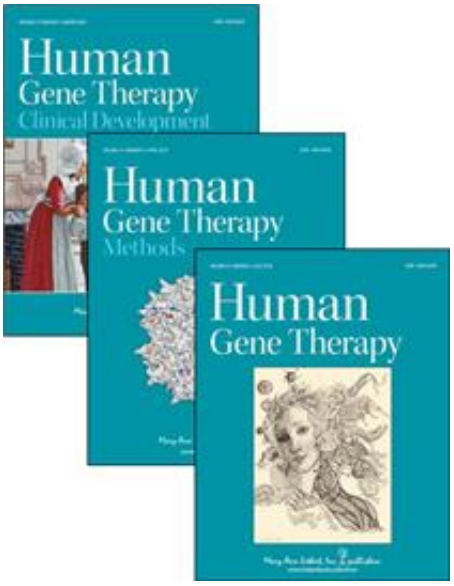

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The encouraging results of early stage clinical studies and the tremendous amount of preclinical data demonstrating the feasibility and promise of gene therapy to treat disorders of the central nervous system (CNS) are driving new advances for the treatment of both genetic and acquired neurodegenerative diseases. Recent progress in therapeutic adeno-associated virus (AAV)-mediated gene transfer strategies and prospects for the future are presented in a Review article in *Human Gene Therapy*.

In the article "Adeno-Associated Virus-Based Gene Therapy for CNS Diseases" coauthors Michael Hocquemiller, Laura Giersch, Mickael Audrain, Samantha Parker, and Nathalie Cartier, of Lysogene (Neuilly sur Seine), Université Paris Descartes (Paris), Université Paris-Sud and Université Paris-Saclay (Orsay), and CEA, DSV, I2BM, MIRCen (Fontenay-aux-Roses), France, describe the expanding scope of CNS diseases being targeted with gene therapy. These include lysosomal storage diseases, Alzheimer disease, Parkinson's disease, amyotrophic lateral sclerosis (ALS), and spinal muscular atrophy (SMA). The researchers discuss the rapid advances in the development of new viral vectors and compare the advantages and limitations of different gene therapy delivery strategies to treat CNS dis-orders.

"The global disease burden of neurodegenerative diseases is increasing dramatically with the aging of populations in the developed world," 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. "Gene therapy promises to be a critically important part of the response of the biomedical research community to this enormous public health challenge."

More information: Michaël Hocquemiller et al, Adeno-Associated Virus-Based Gene Therapy for CNS Diseases, *Human Gene Therapy* (2016). DOI: 10.1089/hum.2016.087

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