Will AAV vectors have a role in future novel gene therapy approaches?

20 March 2017

Recombinant adeno-associated virus (rAAV) vectors for delivering therapeutic genes have demonstrated their safety in multiple diseases and clinical settings over the years and are a proven and effective tool that can be used to deliver new gene editing and replacement and genome modification technologies. The combination of more tailored rAAV delivery vectors and new gene knockdown and editing techniques will enable unique approaches to the therapeutic manipulation of gene expression, as described in an article in *Human Gene Therapy*.

In the article entitled "Future of rAAV Gene Therapy: Platform for RNAi, Gene Editing and Beyond, " Paul Valdmanis and Mark Kay, Stanford University (CA), envision the advances in gene therapy that will be achievable using existing and emerging rAAV technology for the safe and efficient delivery of gene editing technologies such as CRISPR-Cas9, RNA interference and gene silencing strategies, and targeted DNA sequences for use in genome engineering.

"Drs. Kay and Valdmanis envision the exciting prospect of using AAV as a platform to deliver a wide array of novel tools for genetic manipulation of the genome and of gene expression," 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: Paul N. Valdmanis et al, Future of rAAV Gene Therapy: Platform for RNAi, Gene Editing, and Beyond, *Human Gene Therapy* (2017). DOI: 10.1089/hum.2016.171

gene.html