Update on advances in gene therapy from National Center for Advancing Translational Sciences

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New initiatives by the National Center for Advancing Translational Sciences (NCATS) to use gene therapy approaches to treat rare diseases and especially promising aspects of gene transfer and gene editing technology, such as adeno-associated viral (AAV) vectors and CRISPR-Cas9 are highlighted in an editorial published in *Human Gene Therapy*.

In the article "Gene Therapy: The View from NCATS," Philip Brooks, N. Nora Yang, and Christopher Austin, National Institutes of Health (NIH), Bethesda, MD, describe NCATS' strategy of focusing on platform approaches that can be readily adapted to develop treatments for multiple diseases. The aim is to identify the most promising methods and support their translation from the laboratory to the clinic so patients can benefit from these novel therapeutic strategies.

Among the recent development in gene therapy that are particularly relevant to NCATS' mission and priorities are major advances in the development of viral vectors to treat rare diseases, including AAV, which offers significant advantages over adenovirus and retrovirus vectors for gene transfer, particularly in regard to safety. The authors also discuss rapid advances in other nucleic acid therapeutics such as antisense oligonucleotide drugs and RNA-based therapeutic agents. New gene editing tools that are generating a lot of attention offer the possibility of targeted gene inactivation or insertion of therapeutic genes without the use of viral vectors.

"We are extremely pleased that the Director of the NCATS has chosen our journal to describe his strategic vision for gene therapy," 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: Philip J. Brooks et al. Gene Therapy: The View from NCATS, *Human Gene Therapy* (2016). DOI: 10.1089/hum.2016.29018.pjb