

Gene therapy for liver disease advancing with the help of adeno-associated viral vectors

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Liver-directed gene therapy delivered using adeno-associated viral (AAV) vectors to treat diseases such as hemophilia have advanced into human testing. The potential for continued technological improvements to expand the therapeutic applications of gene therapy to treat liver disorders and the remaining clinical challenges are examined in a comprehensive review article published in *Human Gene Therapy*.

In the article "Adeno-Associated Virus Gene Therapy for Liver Disease," Lisa Kattenhorn and coauthors from Dimension Therapeutics,

Cambridge, MA, provide historical context for the remarkable progress achieved using this viral particular delivery vector to target therapeutic genes to the [liver](#). The authors describe the preclinical and clinical studies that have led to a better understanding of immune responses to AAV gene therapy. In addition, they explore areas for future development and current challenges, including readministration of AAV [gene therapy](#) and minimizing the risk of hepatocellular carcinoma.

"AAV-based genet therapy to the liver has been a platform for transformational new therapies for genetic diseases such as hemophilia and inborn errors of metabolism," 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. "The review by Dr. Kattenhorn and colleagues provides an excellent overview of the current best knowledge in this area."

More information: Lisa M. Kattenhorn et al, Adeno-Associated Virus Gene Therapy for Liver Disease, *Human Gene Therapy* (2016). [DOI: 10.1089/hum.2016.160](#)

Provided by Mary Ann Liebert, Inc

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