

Gene therapy for the treatment of Huntington's disease

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Gene therapy targeting the messenger RNA (mRNA) of the mutated huntingtin gene (HTT) can provide long-lasting therapeutic benefit in Huntington's disease after a single administration. An adeno-associated virus (AAV) gene therapy containing a primary artificial microRNA (pri-

amiRNA) targeting HTT mRNA is described in the peer-reviewed journal *Human Gene Therapy*.

"The challenge of treating autosomal dominant genetic brain disorders has been very daunting, and this pioneering effort by Voyager Therapeutics provides new hope for patients with one of the most tragic such diagnoses, that of Huntington's disease," says Editor-in-Chief of *Human Gene Therapy* Terence R. Flotte, MD.

Dinah Sah, Ph.D., from Voyager Therapeutics, and coauthors conducted a systematic comparison of pri-amiRNA processing of a number of AAV-pri-amiRNAs across multiple in vitro and in vivo systems, including mice and non-human primates.

The investigators selected an optimized pri-amiRNA "for an AAV [gene therapy](#) that exhibits efficient and precise pri-amiRNA processing, along with potent pharmacological activity for HTT lowering and general tolerability in vivo for the treatment of Huntington's disease."

More information: Wei Wang et al, Efficient and Precise Processing of the Optimized Primary Artificial MicroRNA in a Huntingtin-Lowering Adeno-Associated Viral Gene Therapy In Vitro and in Mice and Nonhuman Primates, *Human Gene Therapy* (2021). [DOI: 10.1089/hum.2021.221](#)

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