Researchers report longest duration of therapeutic gene expression
17 June 2019

A therapeutic gene delivered into the spinal canal of infant rhesus monkeys was still being expressed after nearly 4 years, with no evidence of acute or chronic neuronal toxicity, according to a new study published in Human Gene Therapy.

The article entitled "Safe and Sustained Expression of Human Iduronidase After In-trathecal Administration of Adeno-Associated Virus Serotype 9 in Infant Rhesus Mon-keys" was coauthored by James M. Wilson, MD, Ph.D., University of Pennsylvania, Pe-relman School of Medicine (Philadelphia) and a team of researchers from the University of Pennsylvania, University of West Indies (Kingston, Jamaica), and University of California, Davis School of Medicine and California National Primate Research Center.

Dr. Wilson's group used intrathecal injection to deliver adeno-associated virus 9 (AAV9) vectors carrying the gene for the alpha-I-iduronidase (IDUA) enzyme. This enzyme is deficient in mucopolysaccharidosis type I (MPS I), an inherited lysosomal storage disease also known as Hurler disease.

Based on the safety and long-term potency of AAV9-IDUA delivery via the cerebrospinal fluid demonstrated in this study in newborn monkeys, the authors conclude that the clinical development of this method of gene therapy should be pursued for early-onset severe forms of neuropathic storage diseases such as MPS I.

"The problem of physical delivery of AAV vectors to the appropriate cells remains a significant technical hurdle in gene therapy for disorders affecting the central nervous system," 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. "Studies like this one provide criti-cal information on the feasibility of delivery approaches that could directly translate to human infants suffering from these fatal diseases."

Research reported in this publication was supported by the National Institutes of Health under Award Numbers HL085794 and OD011107. The content is solely the responsibility of the au-thors.
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