Promising results with new gene therapy approach for treating inherited neurodegenerative diseases

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A new gene therapy approach designed to replace the enzyme that is deficient in patients with the inherited neurodegenerative disorders Tay-Sachs and Sandhoff diseases successfully delivered the therapeutic gene to the brains of treated mice, restored enzyme function, and extended survival by about 2.5-fold. The implications of these promising results for developing similar gene therapies for use in humans and for targeting additional brain disorders are discussed in two articles.
published in *Human Gene Therapy*.


Steven Gray, University of North Carolina at Chapel Hill, and Jagdeep Walia, Queen's University (Kingston, Canada), led a team of researchers from SickKids and University of Toronto (Canada), New Hope Research Foundation (North Oaks, MN), and University of Manitoba (Winnipeg, Canada), in the successful development of a specialized adeno-associated virus (AAV) vector designed to deliver a gene coding for portions of the alpha and beta subunits of the enzyme that are defective in the Tay-Sachs and Sandhoff mice, respectively. The novel gene transfer vector, administered intravenously, was able to deliver the therapeutic gene to the brain and spinal cord, the targeted site of action.

"This important proof-of-concept study sheds important information on the optimal design of rAAV vectors for this class of disorders," 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.


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