

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*.

Both studies demonstrate the feasibility and efficacy of gene transfer in preclinical models. The articles are entitled "[Novel Vector Design and Hexosaminidase Variant Enabling Self-Complementary Adeno-Associated Virus for the Treatment of Tay-Sachs Disease](#)", by Karumuthil-Melethil, et al.; and "[Systemic Gene Transfer of a Hexosaminidase Variant Using a scAAV9.47 Vector Corrects GM2 Gangliosidosis in Sandhoff Mice](#)", by Osmon et al.

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.

More information: Subha Karumuthil-Melethil et al. Novel Vector Design and Hexosaminidase Variant Enabling Self-Complementary Adeno-Associated Virus for the Treatment of Tay-Sachs Disease, *Human Gene Therapy* (2016). [DOI: 10.1089/hum.2016.013](https://doi.org/10.1089/hum.2016.013)

Karlaina J.L. Osmon et al. Systemic Gene Transfer of a Hexosaminidase Variant Using an scAAV9.47 Vector Corrects GGangliosidosis in Sandhoff Mice, *Human Gene Therapy* (2016). [DOI: 10.1089/hum.2016.015](https://doi.org/10.1089/hum.2016.015)

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