# Gene therapy for lysosomal storage disease shown to be safe and well tolerated

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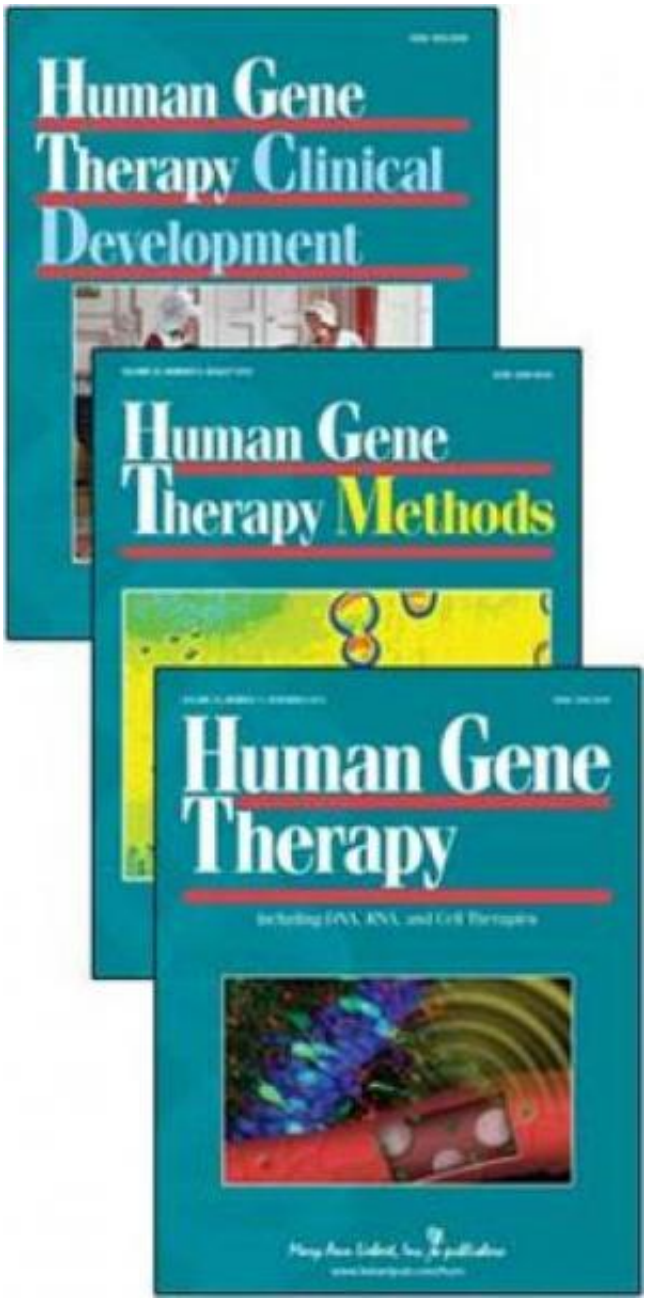

Several young children suffering from a severe degenerative genetic disease received injections of therapeutic genes packaged within a noninfectious viral delivery vector. Safety, tolerability, and efficacy results from this early stage clinical trial are reported in *Human Gene Therapy*.

Marc Tardieu, Université Paris-Sud and INSERM, and a team of international researchers administered the adeno-associated viral (AAV) vector carrying a normal copy of the N-sulfoglycosamine sulfohydrolase (SGSH) gene into the brains of four children affected by mucopolysaccharidosis type IIIA (MPSIIIA), an inherited lysosomal storage disease in which the SGSH gene is defective. The AAV vector also delivered a sulfatase-modifying factor (SUMF1), needed to activate the SGSH protein.

In addition to measures of toxicity, adverse events, and tolerability, the researchers evaluated the children for brain shrinkage (a characteristic of MPSIIIA) and for changes in behavior, attention, sleep, and cognitive benefit. They describe their findings in the article "Intracerebral administration of AAV rh.10 carrying human SGSH and SUMF1 cDNAs in children with MPSIIIA disease: results of a phase I/II trial."

"This is an important new approach for treating CNS manifestations of lysosomal storage diseases that could be applied across a wide array of disorders," says James M. Wilson, MD, PhD, Editor-in-Chief of *Human Gene Therapy*, and Director of the Gene Therapy Program, Department of Pathology and Laboratory Medicine, University of Pennsylvania Perelman School of Medicine, Philadelphia.

**More information:** The article is available on the *Human Gene Therapy* website.

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