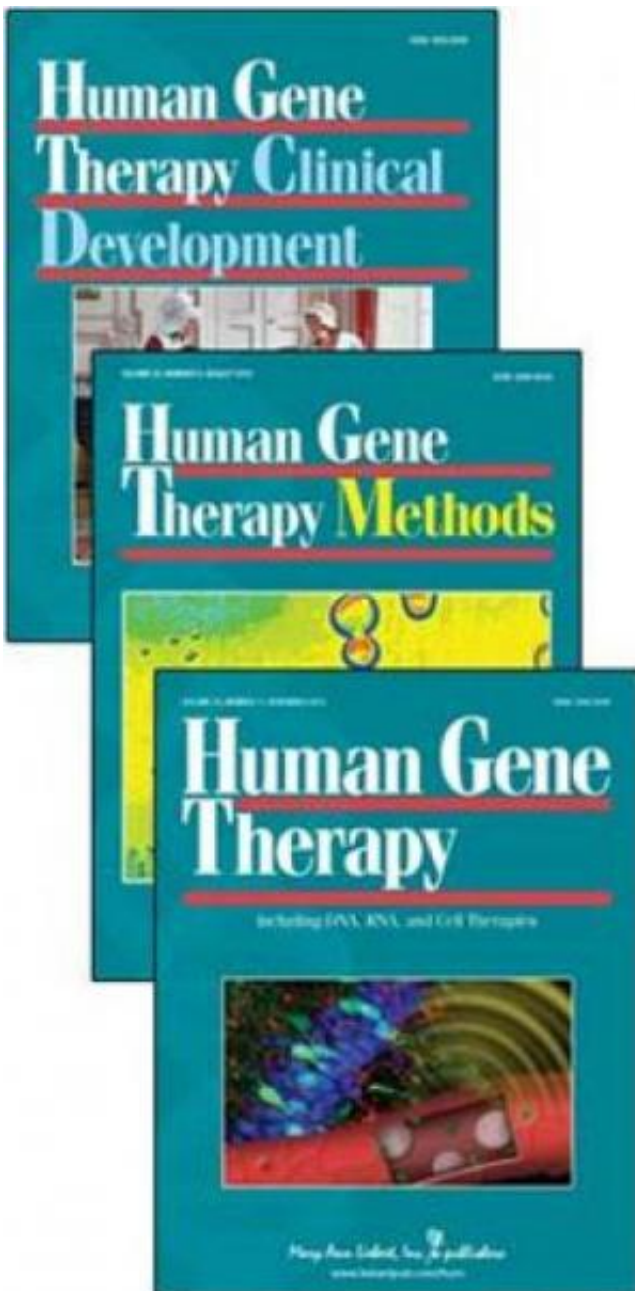


# Gene therapy extends survival in an animal model of spinal muscular atrophy

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To make up for insufficient amounts of SMN protein, the cause of the inherited neuromuscular disease spinal muscular atrophy (SMA), researchers have successfully delivered a replacement SMN1 gene directly to the spinal cords of animal models of SMA. A new study demonstrating that enough copies of the SMN1 gene can be delivered to the spinal cord motor neurons to extend the survival of the treated animals is published in *Human Gene Therapy*.

Marco Passini and coauthors from Genzyme (Framingham, MA), University of California San Francisco, Emory University School of Medicine (Atlanta, GA), and Georgetown University Medical Center (Washington, DC) used an adeno-associated viral vector as the delivery vehicle to transport copies of the SMN1 gene into [motor neurons](#) in the [spinal cord](#) via intrathecal delivery. They report on the effectiveness of restoring the levels of functional SMN protein in normal pig and non-human primate SMA models that would predict efficacy based on gene transfer with the same vector in an authentic mouse model of SMA in the article "[Translational Fidelity of Intrathecal Delivery of Self-Complementary AAV9–Survival Motor Neuron 1 for Spinal Muscular Atrophy](#)."

"This is a very promising and thorough set of preclinical studies that supports rapid translation to the clinic," 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 free on the [Human Gene](#)

*Therapy* website.

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