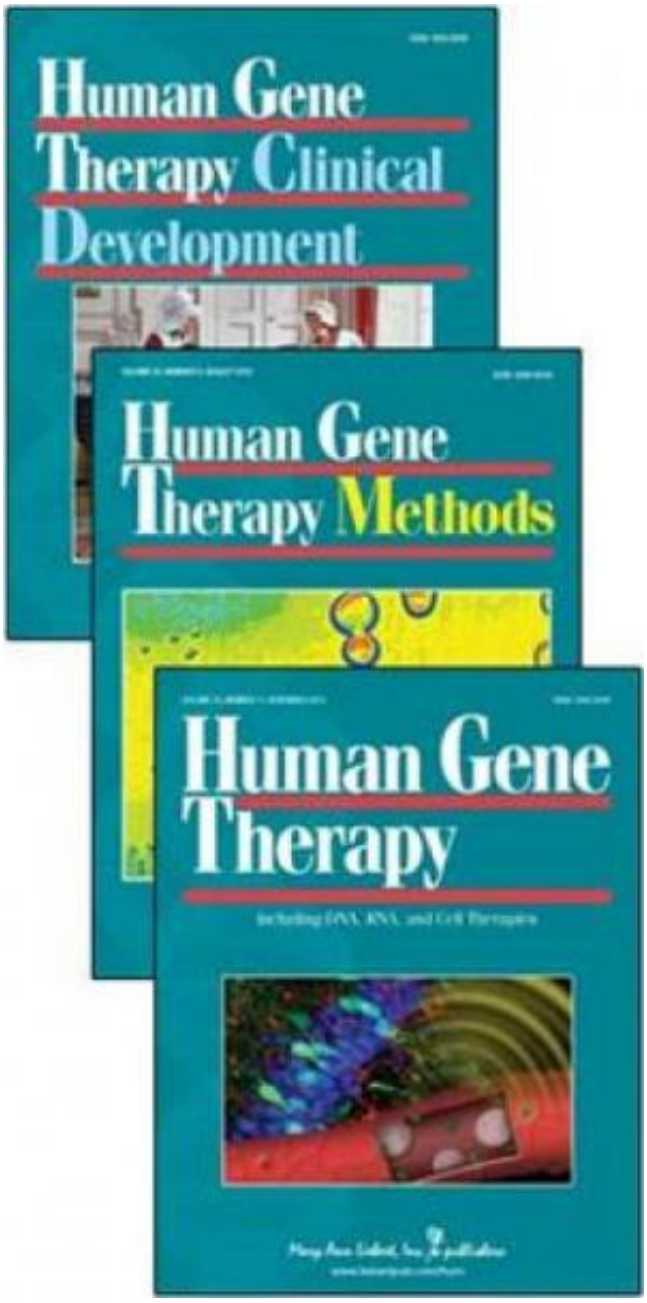
Does the body's immune response to viral vector delivery systems affect the safety or efficacy of gene therapy?

January 8 2014



Packaging replacement genes in viruses is an effective method to deliver them to target tissues, but the human body mounts an immune response against the virus. The systemic and local immune reactions induced by an adeno-associated virus (AAV)-based gene therapy to treat lipoprotein

lipase deficiency, approved for use in Europe, does not affect the safety of gene therapy or expression of the replacement gene for at least one year after delivery, according to a study published in *Human Gene Therapy*.

Valeria Ferreira and coauthors, uniQure BV and Academic Medical Center, Amsterdam, the Netherlands, and University of Montreal and Chicoutimi Hospital, Quebec, Canada, evaluated measures of inflammation and adverse clinical events and the expression of a replacement lipoprotein lipase (LPL) gene that was injected intramuscularly into patients with LPL deficiency. The gene was packaged in an AAV vector, as described in the article "Immune responses to intramuscular administration of alipogene tiparvovec (AAV1-LPLS447X) in a phase II clinical trial of Lipoprotein Lipase deficiency (LPLD) gene therapy."

"The clinical data published in this paper were critical to the approval of Glybera," says James 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. "Furthermore, they provide context for laboratory measurements of immune responses which apparently did not impact product performance."

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

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