

Safety of gene transfer to treat heart failure supports further clinical development

June 6 2017



Credit: Mary Ann Liebert, Inc., publishers

Based on the encouraging safety data that has emerged from multiple clinical trials that used different gene transfer approaches to improve heart function in patients suffering from heart failure with reduced ejection fraction, researchers conclude that this therapeutic strategy can be advanced with acceptable risk. They review the results of completed clinical studies and identify the current challenges in an article published in *Human Gene Therapy*.

In the article entitled "Randomized Clinical Trials of Gene Transfer for

Heart Failure with Reduced Ejection Fraction," William Penny and H. Kirk Hammond, VA San Diego Healthcare System and University of California, San Diego, describe the different genes delivered to the patients and their intended effects, and the two gene delivery methods used in the clinical trials—injection of a virus vector into the heart muscle or delivery of a plasmid to the endocardium, the membrane lining the chambers of the heart. Despite a promising safety profile for [gene transfer](#) in these patients, these early studies yielded mixed results in terms of efficacy, necessitating further development and testing.

"Complex multifactorial diseases like congestive [heart failure](#) represent the greatest challenge faced by clinical gene therapy scientists. There is no substitute for large-scale [randomized clinical trials](#) in the advancement of the field," 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: William F. Penny et al, Randomized Clinical Trials of Gene Transfer for Heart Failure with Reduced Ejection Fraction, *Human Gene Therapy* (2017). [DOI: 10.1089/hum.2016.166](https://doi.org/10.1089/hum.2016.166)

Provided by Mary Ann Liebert, Inc

Citation: Safety of gene transfer to treat heart failure supports further clinical development (2017, June 6) retrieved 20 April 2024 from <https://medicalxpress.com/news/2017-06-safety-gene-heart-failure-clinical.html>

<p>This document is subject to copyright. Apart from any fair dealing for the purpose of private study or research, no part may be reproduced without the written permission. The content is provided for information purposes only.</p>
--