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

6 June 2017

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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

Provided by Mary Ann Liebert, Inc

APA citation: Safety of gene transfer to treat heart failure supports further clinical development (2017, June 6) retrieved 11 May 2021 from https://medicalxpress.com/news/2017-06-safety-gene-heart-failure-clinical.html

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