

# Progress and promise of gene transfer and gene editing to cure beta-thalassemias

5 May 2016

Promising results from the first clinical trials of globin gene transfer to treat beta-thalassemias-inherited forms of anemia-have eliminated the need for blood transfusions in some individuals. Enhancing current gene therapy strategies and applying new gene editing tools to correct beta-globin deficiencies and to reactivate fetal hemoglobin production are among the exciting new advances being pursued in the search for a cure for severe globin disorders, as described in *Human Gene Therapy*. The article is available free for download on the *Human Gene Therapy* website until June 5, 2016.

Jorge Mansilla-Soto, Isabelle Riviere, Farid Boulad, and Michel Sadelain, Memorial Sloan Kettering Cancer Center, New York, NY, are coauthors of the article "[Cell and Gene Therapy for the Beta-Thalassemias: Advances and Prospects](#)." They present the rationale for using globin gene therapy to cure beta-thalassemias and discuss the four Phase I clinical studies underway in the U.S. and Europe. The authors also highlight the important role that emerging gene editing and gene repair technologies can have in correcting beta-globin deficiencies. The possibility for a genetic intervention to lead to a cure for the beta-thalassemias is increasingly likely the researchers conclude.

This article is part of a Festschrift in honor of George Stamatoyannopoulos, MD, DrSci, Professor of Medicine and Genome Sciences, and Director, Markey Molecular Medicine Center, University of Washington, Seattle.

"The review from the Sadelain lab highlights the tremendous progress in [gene therapy](#) for hemoglobin disorders," 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. "It is very fitting that it was authored by such a

prominent translational physician-scientist who was inspired by Dr. Stam."

**More information:** Jorge Mansilla-Soto et al, Cell and Gene Therapy for the Beta-Thalassemias: Advances and Prospects, *Human Gene Therapy* (2016). [DOI: 10.1089/hum.2016.037](#)

Provided by Mary Ann Liebert, Inc

APA citation: Progress and promise of gene transfer and gene editing to cure beta-thalassemias (2016, May 5) retrieved 11 May 2021 from <https://medicalxpress.com/news/2016-05-gene-beta-thalassemias.html>

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