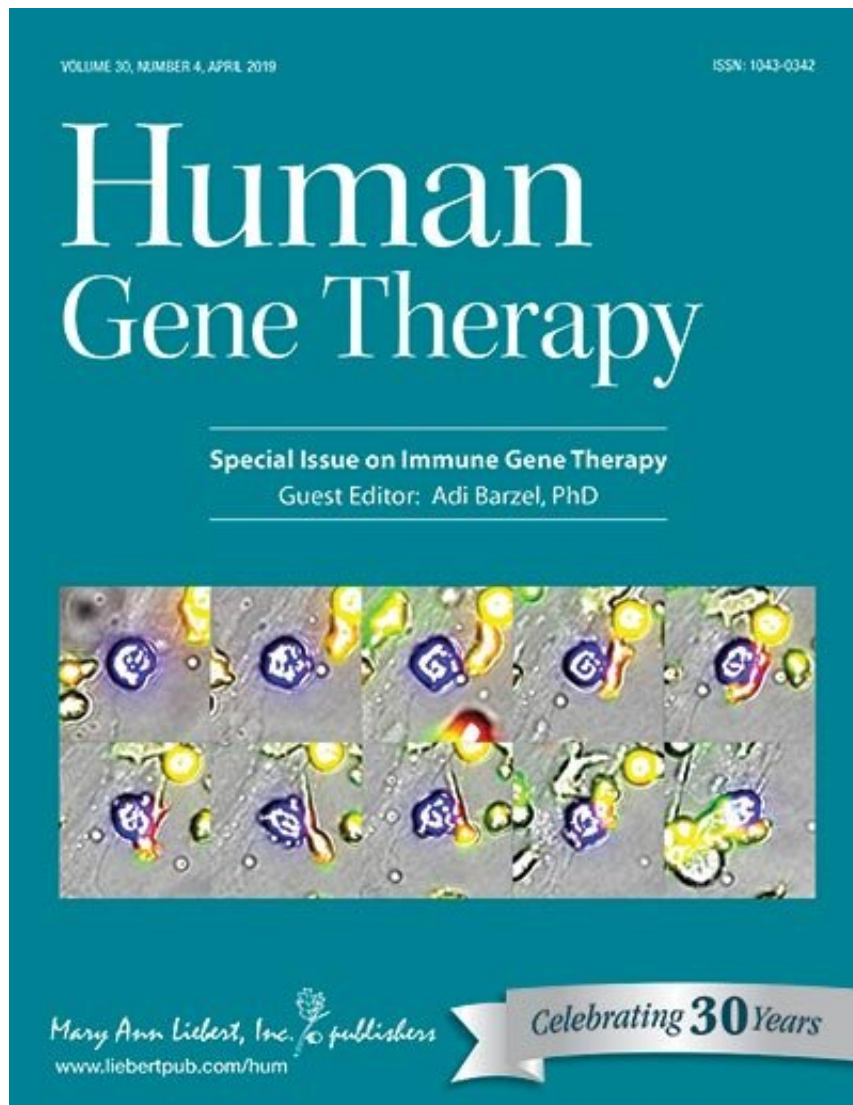


# Estimating the efficacy and cost of curative gene therapy for beta-thalassemia

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Credit: Mary Ann Liebert, Inc., publishers

Gene therapy offers the promise of a cure for beta-thalassemia and a new study has shown that it is associated with fewer complications and hospital admissions over 2 years than treatment by allogeneic hematopoietic stem cell transplantation (HSCT). The study, which analyzes and compares the effectiveness and cost of gene therapy versus (HSCT) in patients with major beta-thalassemia is published in *Human Gene Therapy*.

Séverine Coquerelle, URC Eco-Assistance Publique Hôpitaux de Paris, Université Paris Diderot (Sorbonne Paris Cité), and CRESS, INSERM UMR (Paris), France led a team of French researchers in publishing the article entitled "Innovative Curative Treatment of Beta Thalassemia: Cost-Efficacy Analysis of Gene Therapy Versus Allogeneic Hematopoietic Stem-Cell Transplantation." Patients treated with HSCT had 3 times more frequent infectious complications. Gene [therapy](#) was shown to be about 2.8 times more costly, with nearly half the cost of gene therapy accounted for by preparation of the delivery vector.

"There has been much discussion and controversy about the high cost of gene therapy, but what has been lacking is a direct comparison to [alternative therapies](#), which themselves are also often very costly and may produce suboptimal outcomes," 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. "This study does a critical comparison of both cost and outcomes for patients with beta-thalassemia, whose primary alternative to gene therapy would be hematopoietic stem cell transplantation. These data may enable a more rational debate of the overall value of [gene therapy](#) for this relatively common genetic disease."

**More information:** Séverine Coquerelle et al, Innovative Curative Treatment of Beta Thalassemia: Cost-Efficacy Analysis of Gene

Therapy Versus Allogenic Hematopoietic Stem-Cell Transplantation, *Human Gene Therapy* (2019). [DOI: 10.1089/hum.2018.178](https://doi.org/10.1089/hum.2018.178)

Provided by Mary Ann Liebert, Inc

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