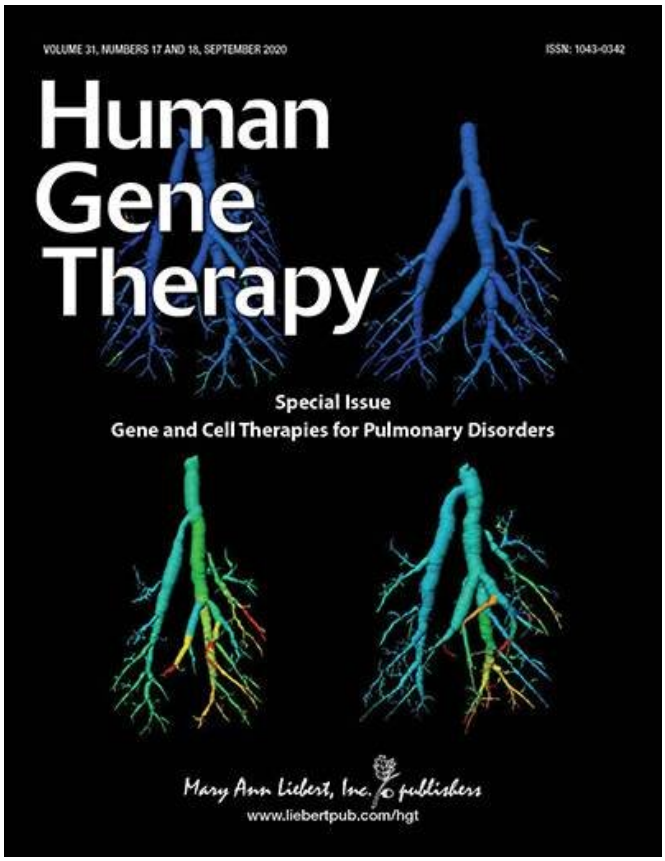
Treating cystic fibrosis with mRNA therapy or CRISPR

9 October 2020



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The potential for treating cystic fibrosis (CF) using mRNA therapies or CRISPR gene editing is possible regardless of the causative mutation. CF clinical trials showing that a genotype-agnostic gene therapy for CF is possible are reviewed in the peer-reviewed journal *Human Gene Therapy*.

"Treating CF by delivering mRNA that encodes CFTR has the potential to work in any CF patient, independent of the underlying mutation," state James Dahlman, Georgia Institute of Technology, and coauthors. "Another potential treatment is utilizing mRNA encoding nucleases such as CRISPRCas9 accompanied by gRNA and using them to edit DNA in target cells."

Challenges remain to be able to utilize these approaches successfully. First among them is the need to identify drug delivery systems that can reach pulmonary epithelial cells at low doses.

"CF was the first disease target in humans for several vector platforms, including rAAV and rAd. It is gratifying to see these newer technologies applied to CF, particularly to the 5% of patients whose mutations are resistant to CFTR modulator drugs," according to Editor-in-Chief of Human Gene Therapy Terence R. Flotte, MD, Celia and Isaac Haidak Professor of Medical Education and Dean, Provost, and Executive Deputy Chancellor, University of Massachusetts Medical School.

More information: Alejandro Da Silva Sanchez et al. Treating Cystic Fibrosis with mRNA and CRISPR, *Human Gene Therapy* (2020). DOI: 10.1089/hum.2020.137

Provided by Mary Ann Liebert, Inc

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