Novel therapeutic strategy for single gene disorders delivers RNA that encodes the missing protein

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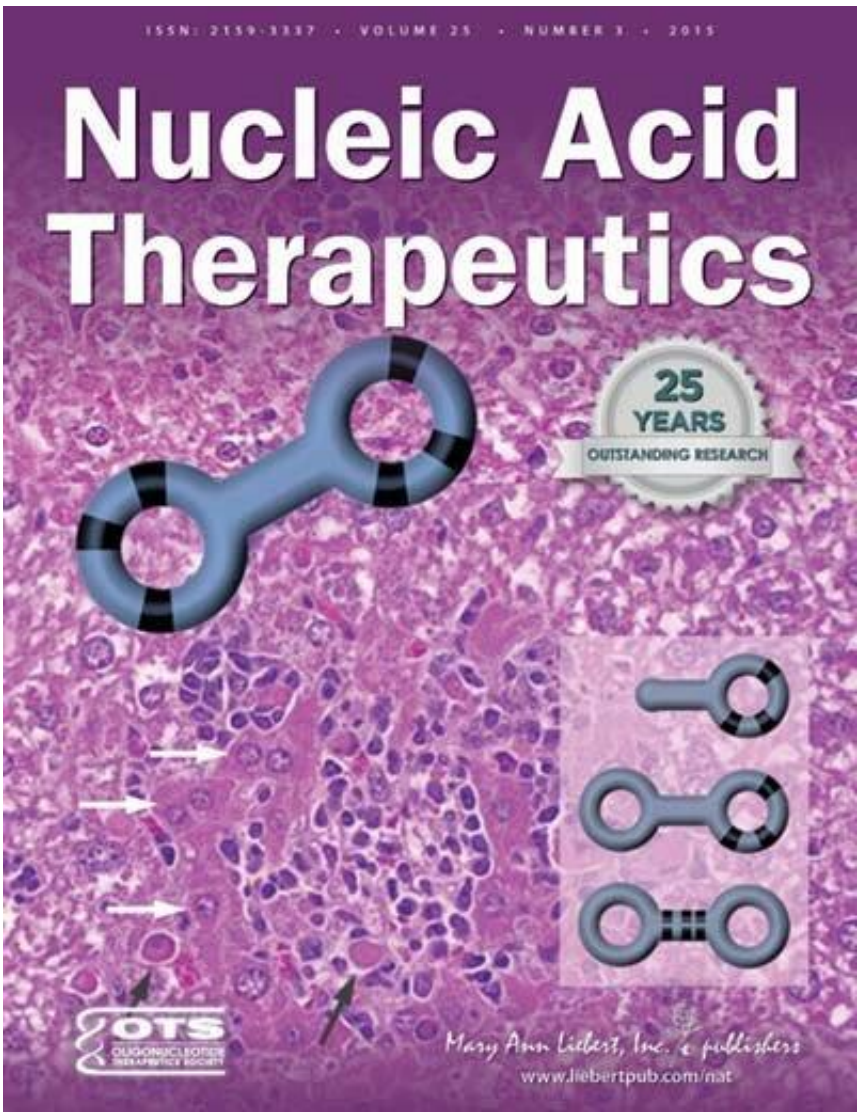

Credit: Mary Ann Liebert, Inc., publishers

Researchers have demonstrated the feasibility of delivering an RNA that encodes for the protein alpha-1-antitrypsin (AAT)—which is missing or nonfunctional in the genetic disorder AAT deficiency—into cells in the laboratory, enabling the cells to produce highly functional AAT. This innovative approach to treating single gene disorders such as AAT deficiency offers and safe, simpler, and more cost-effective alternative to gene therapy or protein replacement, according to the authors of the study published in *Nucleic Acid Therapeutics*.

In the article "In vitro Evaluation of a Novel mRNA-Based Therapeutic Strategy for the Treatment of Patients Suffering from Alpha-1-Antitrypsin Deficiency", Tatjana Michel, Stefanie Krajewski, and coauthors, University Medical Center, Tuebingen, Germany, produced a messenger RNA sequence that cells can translate to generate the AAT protein. The researchers assessed the stability and utility of the encapsulated RNA over time and evaluated the amount of AAT protein produced by the cells and how well the protein functioned. The data show no negative effects of the transfected RNA on the viability of the cells and no immune activation.

"The field is looking for advances in modified mRNA as a therapeutic strategy," says Executive Editor Graham C. Parker, PhD, The Carman and Ann Adams Department of Pediatrics, Wayne State University School of Medicine, Children's Hospital of Michigan, Detroit, MI. "Demonstrations such as this from the University Medical Center, Tuebingen, Germany, show real progress."

More information: The article is available free on the *Nucleic Acid Therapeutics* website until August 27, 2015.