Targeted antisense oligonucleotide drug tested in humans

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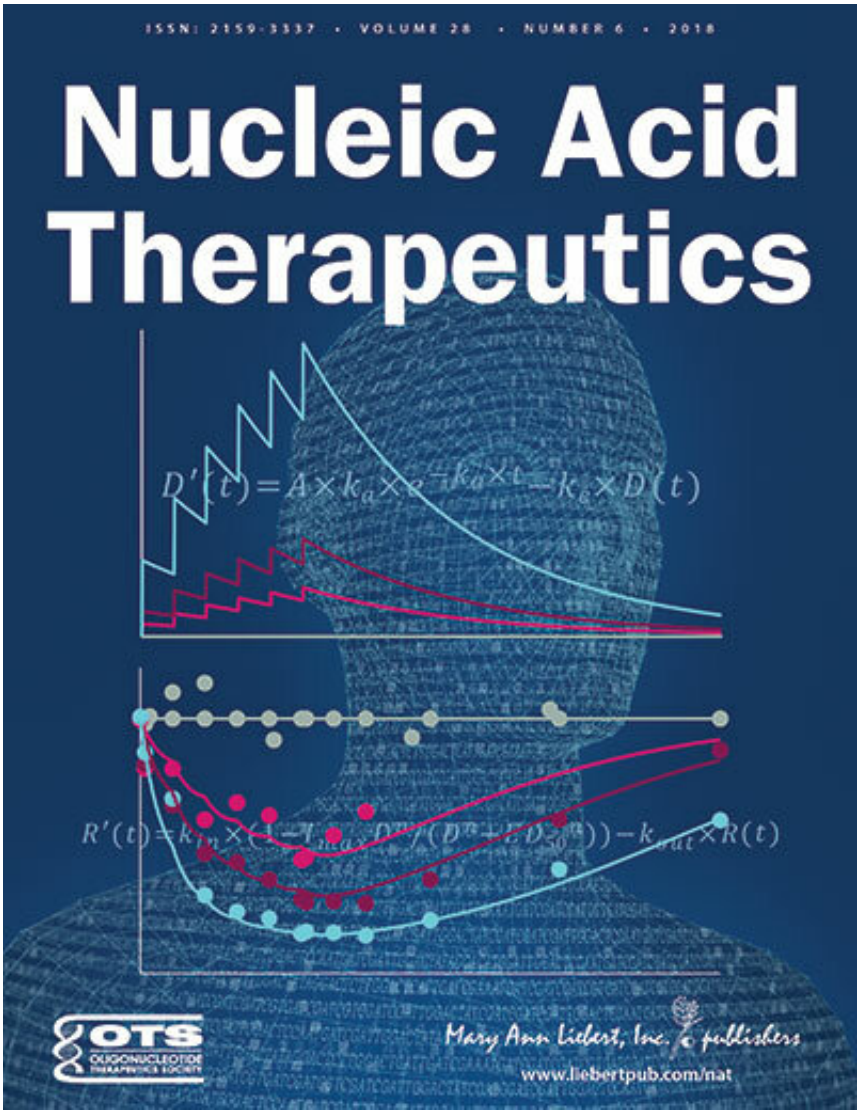

Credit: Mary Ann Liebert, Inc., publishers

A first-in-human study with a new class of antisense oligonucleotide therapeutics showed the ability to target the RNA-silencing drug to the liver, resulting in improved potency and safety at therapeutic doses. The design and results of this trial, conducted in healthy human volunteers are reported in *Nucleic Acid Therapeutics*.

Stanley Crooke and a team of researchers from Ionis Pharmaceuticals, Carlsbad, CA coauthored the article entitled "Integrated Assessment of the Clinical Performance of GalNAc3-Conjugated 2'-O-Methoxyethyl Chimeric Antisense Oligonucleotides: 1. Human Volunteer Experience." They assessed the safety profile of an antisense oligonucleotide to which had been added a new type of chemical conjugate, N-acetylgalactosamine, or GalNAc3, which selectively targets the systemically administered drug for uptake by the liver. They reported that the conjugated drug was up to 30-fold more potent than the parent antisense oligonucleotide that lacked GalNAc3.

"We applaud and encourage the continued willingness to share such valuable human safety data," says Executive Editor Graham C. Parker, Ph.D., The Carman and Ann Adams Department of Pediatrics, Wayne State University School of Medicine, Children's Hospital of Michigan, Detroit, MI.

More information: Stanley T. Crooke et al, Integrated Assessment of the Clinical Performance of GalNAc3-Conjugated 2′-O-Methoxyethyl Chimeric Antisense Oligonucleotides: I. Human Volunteer Experience, *Nucleic Acid Therapeutics* (2018). DOI: 10.1089/nat.2018.0753