

Targeted antisense oligonucleotide drug tested in humans

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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, Nacetylgalactosamine, or GalNAc3, which selectively targets the systemically administered drug for uptake by the liver. They reported that the conjugated <u>drug</u> was up to 30-fold more potent than the parent antisense <u>oligonucleotide</u> that lacked GalNAc3.

"We applaud and encourage the continued willingness to share such valuable human <u>safety</u> 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

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