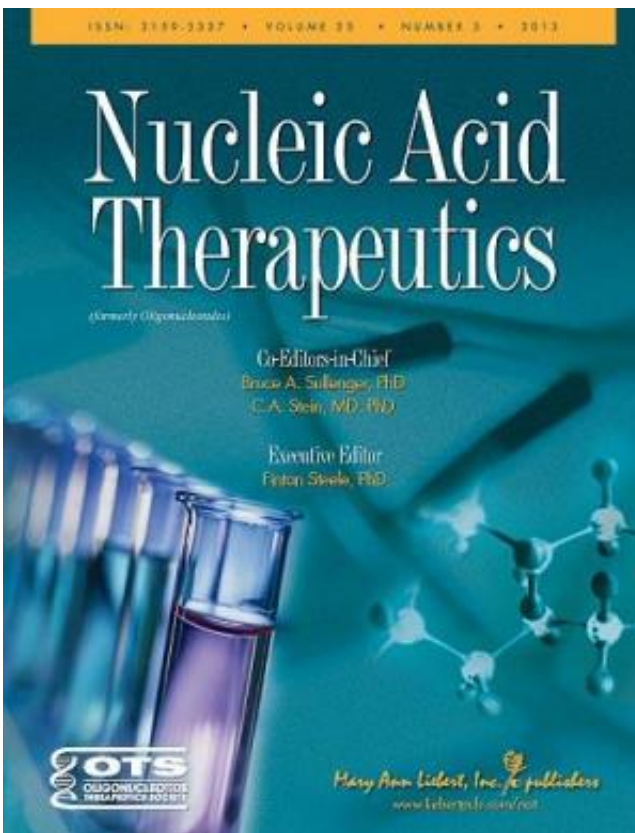


New study shows a breadth of antisense drug activity across many different organs

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Antisense therapeutics, a class of drugs comprised of short nucleic acid sequences, can target a dysfunctional gene and silence its activity. A new study has shown that antisense drugs delivered systemically show activity in a wide range of tissues and organs, supporting their broad therapeutic

potential in many disease indications, as described in an article in *Nucleic Acid Therapeutics*.

Gene Hung, Xiaokun Xiao, Raechel Peralta, Gourab Bhattacharjee, Sue Murray, Dan Norris, Shuling Guo, and Brett Monia, Isis Pharmaceuticals, Carlsbad, CA, developers of antisense therapeutics, compared two antisense drug chemistries (Generation 2.0 and 2.5) designed to target a gene that is expressed by virtually all cells in mice and non-human primates. They demonstrated antisense activity in many tissues and cell types, including liver, kidney, lung, muscle, adipose, adrenal gland, and peripheral nerves. The Generation 2.5 antisense compound was more effective in a wider range of tissues, according to the results presented in the article "[Characterization of Target mRNA Reduction Through In Situ RNA Hybridization in Multiple Organ Systems Following Systemic Antisense Treatment in Animals.](#)"

"This seminal work addresses one of the most important questions facing the field, the demonstration and evaluation of multiple organ targeting by *Nucleic Acid Therapeutics*," 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. "This publication provides a benchmark for convergent analyses in multiple models for preclinical efficacy evaluation."

Nucleic Acid Therapeutics is under the editorial leadership of Co-Editors-in-Chief Bruce A. Sullenger, PhD, Duke Translational Research Institute, Duke University Medical Center, Durham, NC, and C.A. Stein, MD, PhD, City of Hope National Medical Center, Duarte, CA; and Executive Editor Graham C. Parker, PhD.

More information: The article is available on the *Nucleic Acid Therapeutics* [website](#).

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