Nanoparticles that deliver oligonucleotide drugs into cells described

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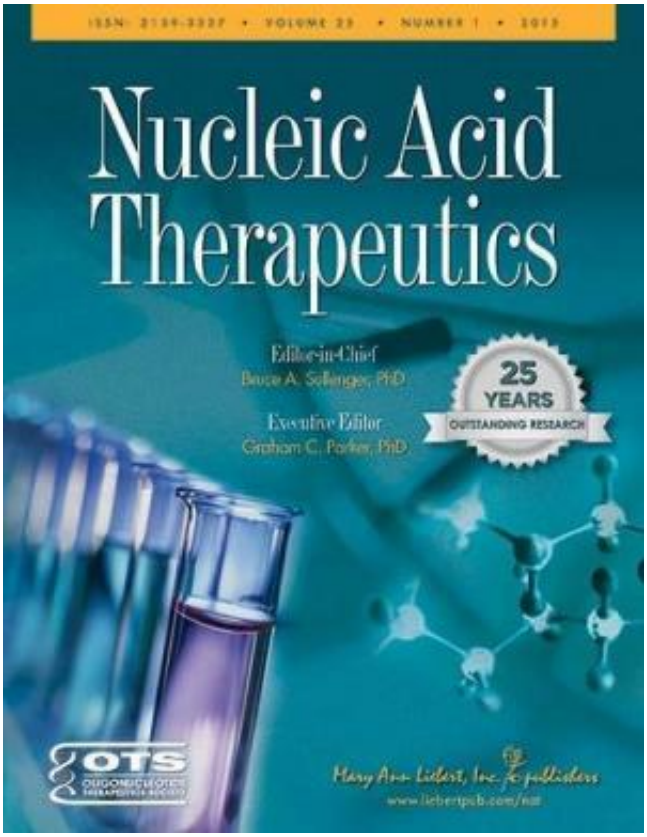

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Therapeutic oligonucleotide analogs represent a new and promising family of drugs that act on nucleic acid targets such as RNA or DNA; however, their effectiveness has been limited due to difficulty crossing the cell membrane. A new delivery approach based on cell-penetrating

peptide nanoparticles can efficiently transport charge-neutral oligonucleotide analogs into cells, as reported in *Nucleic Acid Therapeutics*.

In the article, "Peptide Nanoparticle Delivery of Charge-Neutral Splice-Switching Morpholino Oligonucleotides," Peter Järver and coauthors, Cambridge Biomedical Campus (U.K.), Karolinska University Hospital (Huddinge, Sweden), Stockholm University (Sweden), Alexandria University (Egypt), and University of Oxford (U.K.), note that while delivery systems exist to facilitate cell entry of negatively charged oligonucleotide drugs, these approaches are not effective for charge-neutral oligonucleotide analogs. The authors describe lipid-functionalized peptides that form a complex with charge-neutral morpholino oligonucleotides, enabling them to cross into cells and retain their biological activity.

"The exploitation of phosphorodiamidate morpholinos represents an exciting approach to treating a number of therapeutic targets," 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 paper suggests an intriguing but practical approach to solving the lack of a convenient non-covalent delivery system."

More information: The article is available free on the *Nucleic Acid Therapeutics* website.

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https://phys.org/news/2015-01-nanoparticles-oligonucleotide-drugs-cells.html