Packaging and Delivering Nucleic Acids for in vivo Applications

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BACKGROUND

Nucleic acids have exceptional potential in the preparation of complex nanostructured materials for use as therapeutic agents and as powerful investigative tools. However, unmodified nucleic acids are inherently susceptible to enzymatic degradation in biological milieu, limiting their practical utility in detection and as therapeutics in real world applications. Specifically, new strategies are needed for the preparation of well-defined, stable and competent nucleic acid-based materials.

TECHNOLOGY DESCRIPTION

UC San Diego researchers have developed a novel approach for rendering nucleic acids resistant to two key classes of nuclease that are otherwise capable of rapidly degrading substrates in a sequence selective or non-selective fashion.

This technology packs nucleic acids into polymeric supramolecular nanostructures and render them resistant to nuclease digestion while maintaining sequence selective hybridization competency. Predicated on the idea that steric hindrance through dense packaging limits the accessibility of DNA to selective and non-selective nucleases, this morphology allows access to additional complementary DNA strands, thus preserving the inherent sequence-selective binding and recognition properties.

By packaging and delivering nucleic acids for *in vivo* applications while preserving the integrity of the base-sequence exposed to nuclease-rich environments, this technology will facilitate more potent and selective communication with important cellular machinery.

SUGGESTED USES

Biomedical research. Cell cultures or animal models for functional studies on microRNA, gene therapy, experiments to understand basic fundamentals of this area of biology and molecular genetics. Use can be *in vitro* or *in vivo* for manipulations to understand how altering inhibiting genes or microRNA, in specific cells, affects a biological, biochemical, or genetic process.

Therapeutic applications. Clinical trials for targeting microRNA in disease (e.g., Hepatitis C). The compounds and methods described here can target dopamine neurons, which are implicated in e.g., neurodegenerative disease (e.g., Parkinson's disease), addiction, mood disorder, and the like. MicroRNA are clearly implicated in some of the cellular and neuroanatomical processes of these diseases and the new technology described here can be employed for therapy. The targeting ligand can also be adapted for other cell types and diseases in other tissues and organ systems.

Deliver nucleic acids in vivo. This is the key hurdle in gene and nucleic acid therapies. That is, many systems have been proven *in vitro*, but few are amenable to widespread use *in vivo*.

INTELLECTUAL PROPERTY INFO

US Patent rights are available for commercial development

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OTHER INFORMATION

KEYWORDS
DNA, nanotechnology, polymer, nuclease, resistance

CATEGORIZED AS
- **Materials & Chemicals**
  - Biological
  - Nanomaterials
- **Medical**
  - Delivery Systems
  - Diagnostics
- **Nanotechnology**
  - NanoBio
- **Research Tools**
  - Nucleic Acids/DNA/RNA
- **Sensors & Instrumentation**
  - Biosensors
  - Medical

RELATED CASES
2012-207-0

RELATED MATERIALS

▶ Rush AM, Thompson MP, Tatro ET, Gianneschi NC. Nuclease-resistant DNA via high-density packing in polymeric micellar nanoparticle coronas. ACS Nano. 2013 Feb 26;7(2):1379-87. - 02/26/2013

PATENT STATUS

Country	Type	Number	Dated	Case
United States Of America	Issued Patent	10,046,057	08/14/2018	2012-207

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