

## Targeted Nanoparticles for the Treatment of Virus-infected or Cancerous Cells

Ref. No. E-039-2012

**Keywords:** Therapeutic, cancer, viral infections, RNA nanoparticles, siRNA.

**Summary:** The National Cancer Institute seeks parties interested in collaborative research to co-develop and commercialize therapeutic RNA/DNA nanoparticles.

**Technology:** Current treatments for cancer and viral infection are limited remedies that often suppress cell or viral replication rather than eliminate diseased cells entirely from the body. A further limitation is that these therapies often compromise healthy cells as well, leaving problems of recurrence and side effects.

Researchers at NCI's [Nanobiology Program](#) developed a novel therapeutic nanoparticle (NP) system harboring therapeutic small siRNA that can significantly enhance effectiveness and specificity of treatments by killing diseased cells.

Nanoparticles attached to RNA/DNA hybrids encode recognition sites for target genes and partial siRNA sequences of human anti-apoptotic genes. Individually, each of the hybrids is functionally inactive and functional representation can only be activated by the re-association of at least two cognate hybrids simultaneously present in the same cell. Overall, this novel approach allows each NP to have recognition sites for different target genes (e.g. viral genes in viral infection, abnormally regulated genes in cancer), providing versatile options for selecting cells to kill with far greater specificity. Besides therapeutic siRNA, RNA/DNA hybrids on NPs can encode fluorescent markers to specifically visualize the diseased cells.

### Potential Commercial Applications:

- Therapeutic siRNA for cancer and viral infections
- Diagnostic to visualize cancerous or virus-infected cells or track delivery and effectiveness of siRNA treatment.
- Research tool to study cancer, viral infection, or other diseases

### Competitive Advantages:

- Novel way for multiple functionality delivery and activation
- Enhanced chemical stability and pharmacokinetics due to the average size of nanoparticles exceeding 10nm
- Increased specificity for selecting cells of interest using more than one target gene

**Development Stage:** Preclinical, *in vivo* animal data available

**Patent Status:** US Provisional Application No. 61/561,257 filed 17 Nov 2011.

**Related technology:** NIH Ref. # E-038-2012/0 U.S. Patent Application No. 61/561,247 filed 17 Nov 2011

### Publications:

1. Afonin KA et al, Co-Transcriptional Assembly of Chemically Modified RNA Nanoparticles Functionalized with siRNAs. *Nano Lett.* 12: 5192-5195, 2012 [[PMID 23016824](#)]
2. Grabow W et al, Self Assembling RNA Nanorings Based on RNA I/II Inverse Kissing Complexes *Nano Lett* 11: 878-87, 2011 [[PMID 21229999](#)]

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3. Afonin KA et al, Design and Self-assembly of siRNA-functionalized RNA nanoparticles for use in automated nanomedicine *Nat Protoc.*6: 2022-34, 2011 [[PMID 22134126](#) ]

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