

Selective Treatment of Cancer Cells, HIV and Other RNA Viruses

Ref. No. E-038-2012

Keywords: Therapeutic, cancer, HIV, viral infections, RNA switches, RNAi.

Summary: The National Cancer Institute seeks parties interested in collaborative research to co-develop therapeutic RNA switches.

Technology: Targeted therapy in cancer or viral infections is a challenge because the disease state manifests itself mainly through differences in the cell interior, for example in a form of the presence of a certain RNA or protein in the cytoplasm.

This technology, developed at the NCI [Nanobiology Program](#), consists of designed RNA switches that activate the RNA interference pathway only in the presence of a trigger RNA or DNA to which they bind, in order to knock down a chosen gene that is not necessarily related to the initial trigger.

This new approach can lead to a new type of drug that has the unique feature of selectively causing a biochemical effect (such as apoptosis) in cells that are infected by RNA viruses (such as HIV), as well as cancer cells. The RNA switch concept can be expanded to selectively treat other genetically related diseases.

Potential Commercial Applications:

- Targeted therapeutic for viral infections, cancer stem cells, and genetically related diseases
- Research tool to study cancer or viral infection

Competitive Advantages:

- Fewer side effects because the therapeutic RNA-interference pathway is only activated by the RNA switch when it is intact and in its active conformation.
- Selectively kills cells infected by RNA viruses
- Contains a minimal number of single stranded nucleotides, thus minimizing the effects of nucleases

Development Stage: Discovery, *in vitro* data available

Patent Status: US Provisional Application No. 61/561,247 filed 17 Nov 2011.

Related technology: NIH Ref. # E-039-2012/0 U.S. Patent Application No. 61/561,257 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](#)]
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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