

## **RNA Interference Sponge Particle**

Technology #14743

### **Applications**

RNAi sponge particle (RNAi-SP) technology can be used in a variety of therapeutic settings where siRNA mediated-gene silencing is a viable treatment strategy.

### **Problem Addressed**

In recent years, RNA interference (RNAi) has gained interest as a powerful tool for suppressing gene expression. RNAi involves the intracellular delivery of double stranded RNAs (dsRNAs) which have sequences that target a gene of interest. Once inside the cell, these dsRNAs activate the RNA interference pathway to silence their target genes. The ability to safely deliver stable RNA into the cell continues to be a key challenge in the nascent field, and a new method such as RNAi-SP has the potential to greatly advance RNAi therapeutics.

### **Technology**

This technology takes advantage of rolling circle transcription (RCT), an artificial nucleotide replication method, to synthesize concatemerized strands of cleavable RNA. The concatemerized RNA strands self-assemble into therapeutically deliverable sponge-like particles with a diameter of approximately 2  $\mu\text{m}$ . The RNA is protected from degradation because it forms double-stranded structures within these particles. These nanoparticles can be reduced to a 200 nm diameter size through treatment with cationic polyethylenimine (PEI). Each particle contains approximately a half a million of cleavable RNA strands, which makes their delivery efficiency far superior to currently existing transfection methods. When these cleavable strands are introduced into the cell, they activate the RISC (RNA-induced silencing complex) pathway to knock down intracellular production of any proteins of interest. Test trials on T22 cells have shown that RNAi-SPs can achieve the same knockdown efficiency as siRNA packaged by traditional liposome-based delivery methods at concentrations that are roughly three orders of magnitude less. Furthermore, the cells showed approximately 100% viability, indicating that the RNAi-SP delivery method exhibits low cytotoxicity. *In vitro* injection of RNAi-SP in mice have shown similar results.

### **Advantages**

- New route for effective, highly efficient delivery of siRNA
- Ability to generate large amounts of siRNA in a form that assembles directly into a drug carrier that can be used for direct transfection
- siRNA is protected within sponge particle in the form of double-stranded polymeric RNAi
- Broad clinical applications with RNA interference

### **Categories For This Invention:**

Life Sciences

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Research Tools

Micro/nanoparticles (Research Tools)

RNA

Therapeutics

Gene Silencing

## Intellectual Property:

Nucleic acid particles, methods and use thereof

Issued US Patent

9,737,557

## Inventors:

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## Publications:

Self-Assembled RNA Interference Microsponges for Efficient siRNA Delivery

Nature Materials

Feb 26, 2012, p. 316-322

## External Links:

Hammond Lab

<https://hammondlab.mit.edu/>

## Image Gallery:

