

## Sustained Intracellular RNA Delivery and Expression

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### SUMMARY

UCLA researchers in the Department of Chemistry and Biochemistry have developed a novel method for high protein expression levels, in situ, involving RNA-based therapeutics.

### BACKGROUND

The RNA therapeutics industry is growing at a CAGR of 12% and is expected to reach \$1.2 billion by 2020. These are a novel class of therapeutics, currently in clinical trials for cancer, immunotherapy and vaccination. An RNA therapeutic is essentially an mRNA that codes for any gene of interest, such as an antibody or an immune modulatory protein. Upon delivery of the mRNA, the cellular machinery initiates production and thus provides an in vivo synthesis of the therapeutic. However, there are a number of challenges confronting clinical applications of this technology. The instability of RNA and reduced longevity of its effects requires that patients receive regular doses for long-term effects.

### INNOVATION

UCLA researchers have developed a novel approach for sustained RNA expression in vivo. Using their own technology of artificial and non-infectious virus-like particles, they have developed a method to deliver any gene of interest. Their novel approach can yield 100-1000 times higher levels of production of the gene of interest than delivery of mRNA. It can be used for therapeutic delivery, imaging purposes, as a vaccine platform, as well as for research purposes. The key idea involves the protection of two RNA molecules in identical *but separate* virus-like particles – one containing the gene of interest, and the other containing the gene for an RNA replicase that will strongly amplify the gene of interest.

### APPLICATIONS

- ▶ Sustained expression of large proteins such as antibodies for passive immunization
- ▶ Sustained expression of any therapeutics
- ▶ Research tool for expression of any desired gene in vivo
- ▶ Immunomodulation for cancer therapy

### ADVANTAGES

- ▶ Simple approach compatible with any gene of interest involving in vitro syntheses from purified components
- ▶ Can be used for expressing large genes and proteins such as antibodies

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### INVENTORS

- ▶ Gelbart, William M.

### OTHER INFORMATION

#### KEYWORDS

RNA, Virus, RNA Replication, Gene delivery, therapeutic delivery, transfection

#### CATEGORIZED AS

- ▶ **Medical**
  - ▶ Delivery Systems
  - ▶ Research Tools
  - ▶ Therapeutics
- ▶ **Research Tools**
  - ▶ Expression System
  - ▶ Nucleic Acids/DNA/RNA

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