

FIERCE INNOVATION AWARDS 2016

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Life Science

INNOVATION REPORT

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mRNA WILL QUICKLY CATCH UP--THE TARGETS ARE UNLIMITED

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Although mRNA-based therapies have caught people’s attention in the last few years as vaccines for cancer and infectious diseases, recent announcements suggest mRNA is gaining traction in protein replacement therapy too. Arcturus Therapeutics Inc. has disclosed mRNA therapeutic programs based on delivery systems that produce high protein expression in animal models and hold promise for low toxicity, but the biggest hurdles will be targeting the relevant tissues and sustaining mRNA levels long enough to create viable therapeutics.

Arcturus is basing its mRNA platform on the premise that extending the molecules’ persistence in the plasma, in part by reducing their toxicity, would present a competitive platform and build on its siRNA expertise. Arcturus’ LUNAR™ (Lipid-enabled and Unlocked Nucleomonomer Agent RNA) delivery system employs a proprietary technologies to achieve potent and safe delivery of all RNA modalities. The LUNAR delivery system is completely differentiated from other competing technologies. For example whereas most formulations have dose-limiting toxicity that caps delivery at about 3 mg/kg, Arcturus’ technology allows for the safe delivery of siRNA and mRNA such as levels ≥ 30 mg/kg. Arcturus has also used different LUNAR formulations to deliver mRNA for a variety of proteins *in vitro* and *in vivo* to the liver (both hepatocytes and stellate cells), lung and tumors.



THERE ARE FIVE KEY ISSUES IN THE FIELD OF MESSENGER RNA THERAPEUTICS

- 1) Limited ‘composition of matter’ IP around the API mRNA
- 2) Insufficient delivery of mRNA, especially in primates
- 3) Insufficient stability of nanoparticles that contain mRNA
- 4) Short half-life of mRNA after it is delivered into the target cell
- 5) Decreased potency after repeat dosing observed

ARCTURUS HAS ADDRESSED ALL OF THE ABOVE ISSUES

- 1) UNA Oligomer™ (Unlocked Nucleomonomer Agent):
 - mUNA (UNA modified mRNA) chemistry—composition of matter IP provisional applications submitted
- 2) LUNAR: effective delivery of multiple mRNA constructs in NHPs
- 3) LUNAR-mRNA particles are stable utilizing a trade secret process with stabilizing excipients
- 4) mUNA: UNA modified mRNA (mUNA) significantly improves longevity/AUC compared to conventional mRNA
- 5) LUNAR delivery provides consistent potency after repeat dosing

The next step is to advance LUNAR technology into a human trial, expected to begin this year with the goal of demonstrating safety, tolerability and biological proof of concept. Arcturus has completed its pre-investigational new drug application meeting with the FDA and gained alignment with the agency on its clinical plan.

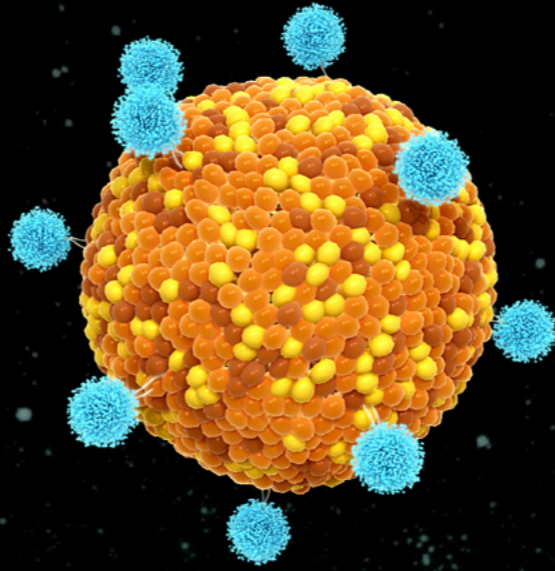


TABLE OF CONTENTS:

- Introduction
- Recognition of Best In Show Winners
- Recognition of Category Winners
- Recognition of Finalists
- Recognition of Judges

STATE OF THE INDUSTRY ARTICLES:

- Biotech Innovation Best in Show: Recursion Pharmaceuticals // Computationally Intelligent Phenotype Screening Platform
- Technology Innovation Best in Show: InCrowd // InCrowd MicroTracker
- Data Analytics/Business Intelligence Best in Show: Covance
- Digital Health Solution: Context Media // the Digital Exam Room Wallboard, Infusion Room Tablet and Patient Mobile Connect
- Drug Delivery Technology: Bicycle Therapeutics // Bicycle and Bicycle Drug Conjugates
- Directory Listings



UNA and LUNAR™ Will Revolutionize RNA Medicines



TRANSPORTERS



RECEPTORS



ANTIBODIES



ENZYMES



TALEN



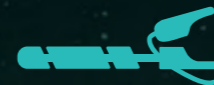
GPCR'S



HORMONES



CHAPERONES



CAR-T



CRISPR