



Arcturus Therapeutics Presents Preclinical Messenger RNA Data in Non-Human Primates at the 2nd International mRNA Health Conference

November 10, 2014

– LUNAR™ delivery of human erythropoietin messenger RNA in non-human primates (N = 3) generated 1000-fold increase of erythropoietin protein with one low dose (0.3 mg/kg)

– Preclinical messenger RNA data in cell and rodent models with cystic fibrosis transmembrane conductance, tumor protein p53, alpha-1 antitrypsin, factor IX, and erythropoietin demonstrate rapid protein production

– Arcturus has now established safe and successful RNA delivery capability in non-human primates for BOTH messenger RNA and siRNA

San Diego, Calif., Nov 10, 2014 – [Arcturus](#) Therapeutics, Inc., a leading RNA medicines company pursuing orphan diseases, today announced it will present a poster at the 2nd International mRNA Health Conference being held on November 11-12, 2014 in Cambridge, MA. The company poster presentation (#13), "Potent Delivery of LUNAR™ Nanoparticles Containing Synthetic mRNA for Therapeutic Protein Expression in Non-Human primates" will take place on the 11th of November, at 7:00 PM EST.

LUNAR™ delivery of synthetic human erythropoietin messenger RNA in non-human primates (N = 3) generated 1000-fold increase of erythropoietin protein with one low dose (0.3 mg/kg). No safety issues were observed in any of the non-human primates indicating LUNAR delivery of messenger RNA was well tolerated. Additional data to be presented include preclinical results from rodent models dosed with therapeutic human messenger RNAs for alpha-1 antitrypsin (AAT), factor IX, and erythropoietin. Delivery of chemically modified messenger RNA in LUNAR™ resulted in rapid expression of protein within 2 hours and lasting for greater than 24 hours. Further, *in vitro* cell culture data with cystic fibrosis transmembrane conductance (CFTR) and tumor protein p53 (p53) messenger RNA will also be included demonstrating the breadth of opportunities for this novel therapeutic approach.

"Messenger RNA offers a powerful way to modulate gene expression within a cell, thereby providing a novel therapeutic approach to treat diseases that were previously inaccessible or relied on expensive and complex protein replacement therapies. We are thrilled to be presenting our messenger RNA data in rodents and non-human primates, showing impressive production of multiple human therapeutic proteins with a large safety margin due to our proprietary LUNAR delivery technology," said Dr. Pad Chivukula, CSO and COO of Arcturus Therapeutics. "UNA chemistry and LUNAR delivery provide the extraordinary flexibility to effectively modulate gene expression in *BOTH* directions – increasing protein expression via messenger RNA and/or decreasing gene activity via small interfering RNA. Arcturus is in a unique position having proven successful RNA delivery capability in non-human primates for *both* messenger RNA and siRNA."

About Arcturus Therapeutics, Inc.

Founded in 2013 and based in San Diego, Arcturus Therapeutics is focused on RNA medicines for the treatment of rare diseases. Arcturus has developed a novel, potent and safe RNA Therapeutics platform called LUNAR™, a proprietary lipid-enabled delivery system for RNA medicines including small interfering RNA, messenger RNA, antisense, and microRNA oligotherapeutics. The company owns Unlocked Nucleic Acid (UNA) chemistry technology and patent portfolio (34 patents, USPTO granted) enabling the targeting of any gene in the human genome. For more information, visit www.ArcturusRx.com.

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