



Arcturus Therapeutics to Present Gene Knockdown Data in Non-Human Primates, Showing up to 94% Reduction in Gene Expression with a Single Low Dose

October 13, 2014

- LUNAR-101, an UNA™ oligomer therapeutic for transthyretin-mediated familial amyloid cardiomyopathy (TTR-FAC), exhibited in non-human primates TTR reduction greater than 90% with a single low dose of 0.3 mg/kg (N = 3).
- Significant TTR reduction is observed even after 40 days; a once-monthly dosing regimen will be clinically investigated in 2015.
- LUNAR-102, an UNA™ oligomer therapeutic for transthyretin-mediated familial amyloid polyneuropathy (TTR-FAP), shows allele selective silencing greater than 100-fold for the V30M variant over the wild-type allele.
- LUNAR™ delivery technology continues to demonstrate an outstanding safety profile; the highest dose tested of 50 mg/kg showed no adverse events.

San Diego, Calif., Oct 13, 2014 – [Arcturus Therapeutics, Inc.](#), a leading RNA medicines company pursuing orphan diseases, today announced it will present a poster at the 10th Annual Meeting of the Oligonucleotide Therapeutics Society on “Exploring Allele-selective siRNAs for Human Transthyretin” (poster number 130) being held October 12-15, 2014 in San Diego, California. The company poster presentation by Pad Chivukula, Ph.D., CSO & COO, will be on the 13th of October, at 4:30 PM PST.

“Arcturus is developing best-in-class RNA medicines using its therapeutic delivery platform LUNAR™ and UNA chemistry. Data demonstrates this promising approach for treating TTR-mediated diseases including allele-selective silencing of the V30M variant,” said Dr. Chivukula. “We believe that the Arcturus platform has broad applications for RNA medicines including breakthrough advances in autosomal dominant disorders.”

Key data presented includes:

- LUNAR-101, an UNA™ oligomer therapeutic targeting transthyretin for the treatment of TTR-mediated cardiomyopathy, demonstrated a 90% reduction in TTR at day 10 and continued reduction at day 20 of 91% in non-human primates (n = 3, up to 94%) after a single low dose of only 0.3 mg/kg.
- The clinical dosing for LUNAR-101 will be investigated with once-monthly intravenous administration coinciding with the patient’s normal physician visit.
- LUNAR-102, an UNA™ oligomer therapeutic targeting transthyretin for the treatment of TTR-mediated polyneuropathy, demonstrated *in vitro* allelic silencing greater than 100-fold for the V30M variant over wild-type allele.
- LUNAR™ delivery technology continues to be safe and well-tolerated; a single high dose of 50 mg/kg in rodents shows no adverse events.

LUNAR™ delivery and UNA oligomer chemistry provide an exciting new approach to treat autosomal dominant diseases where targeting the mutant variant, but not the wild-type allele, is critical for optimal clinical outcomes.

About Transthyretin (TTR)-Mediated Amyloidosis

Transthyretin (TTR)-mediated amyloidosis is a genetically mediated fatal disease caused by mutations in the TTR gene. Mutated TTR, which is mainly synthesized in the liver, causes errant amyloid proteins to aggregate and deposit, destroying body organs and tissue, such as the peripheral nerves and heart. TTR-mediated familial amyloid cardiomyopathy (TTR-FAC) affects approximately 40,000 people with the mean survival of ~2.5 years. TTR-mediated familial amyloid polyneuropathy (TTR-FAP) affects at least 10,000 people. There is a substantial unmet need for effective medicines for patients suffering from TTR-mediated diseases.

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 UNA oligomer 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.

#