# Arcturus Therapeutics, Inc. Announces an RNAi Therapeutic Targeting Transthyretin-mediated Amyloidosis as its Flagship Program

January 13, 2014

**Immediate Release**

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**New Preclinical Results in Non-Human Primates Demonstrate Significant and Sustained Reduction of Transthyretin Using its Proprietary LUNAR™ Delivery Technology**

SAN DIEGO, CA – January 13th, 2014 – Arcturus Therapeutics, Inc., an industry leader in RNA technologies, today announced its flagship program – an RNAi therapeutic targeting transthyretin (TTR)-mediated amyloidosis. Preclinical non-human primate data will be presented at the Biotech Showcase being held January 13, 2014 in San Francisco, California.

Preclinical results from a non-human primate (NHP) study demonstrated > 75% knockdown of serum TTR protein levels after 10 days, with the effect sustained over 3 weeks (0.3 mg/kg, i.v. dosing).

In addition to NHP knockdown data, no adverse events were observed using Arcturus LUNAR ™ delivery technology, in particular, no injection site reactions occurred. Furthermore, a substantial safety window was demonstrated in a multiple dose rat toxicology study – no adverse events observed at the highest doses (30 mg/kg total; administered three times at 10 mg/kg over 15 days). Serum levels of the alanine aminotransferase (ALT) and aspartate aminotransferase (AST) were determined after intravenous administration and no changes in these liver enzymes were observed.

"We are pleased to announce our flagship program targeting TTR including our successful achievement of non-human primate data with durable biological effect and having an impressive safety profile," said Pad Chivukula, Ph.D., Arcturus' CSO and COO. "With this data in hand, we are laser-focused on selecting a wholly-owned clinical drug candidate in 2014 and entering the clinic soon after."

**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, cause errant amyloid proteins to aggregate and deposit, destroying body organs and tissue, such as the peripheral nerves and heart. TTR-mediated polyneuropathy (FAP) affects approximately 10,000 people worldwide and TTR-mediated cardiomyopathy (FAC) affects at least 40,000 people worldwide with the mean survival of ~2.5 years. With no approved therapies, there is a substantial medical need for effective therapeutics for patients suffering from TTR-mediated diseases.

**About Arcturus Therapeutics, Inc.**
Founded in 2013 and based in San Diego, Calif., Arcturus Therapeutics is focused on RNA technologies for the treatment of rare diseases and high unmet medical needs. 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, and microRNA oligotherapeutics. The company owns Unlocked Nucleic Acid (UNA) chemistry technology and patent portfolio (32 patents, USPTO granted) enabling the targeting of any gene in the human genome. For more information, visit www.ArcturusRx.com.

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