



Arcturus Therapeutics Announces Allele-Specific Silencing Technology at IBC's 16th Annual TIDES Conference

May 13, 2014

– *Greater than 100-fold Allele-specific silencing of V30M variant for the treatment of Familial Amyloid Polyneuropathy (TTR-FAP)* –

San Diego, Calif., May 13, 2014 – [Arcturus](#) Therapeutics, Inc., a leading RNA medicines company pursuing rare diseases, today announced the nomination of its second pipeline program targeting the TTR V30M variant using UNATM-mediated RNAi allele-specific silencing for the treatment of TTR-mediated familial amyloid polyneuropathy (FAP). Additional data presented at IBC's 16th Annual TIDES Conference being held on May 12-15 at Rhode Island Convention Center in Providence includes an update on Arcturus' LUNARTM delivery platform demonstrating robust safety in multi-dose rat and single-dose mouse toxicology studies of up to 30 mg/kg showing no change in organ weights, liver enzymes, blood chemistries; supporting a wide therapeutic index.

"We have achieved outstanding V30M variant selectivity of greater than 100-fold utilizing Arcturus UNATM chemistry opening the doors to breakthrough advances in autosomal dominant disorders," said Joseph E. Payne, CEO. "We are enthusiastic about the powerful attributes of UNATM chemistry and the significant safety profile of LUNARTM delivery as we advance and grow our RNA medicines pipeline."

The company presentation by Pad Chivukula, Ph.D., CSO & COO, will be on Wednesday, May 14, 2:30 PM ET.

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 cardiomyopathy (FAC) affects approximately 40,000 people with the mean survival of ~2.5 years. TTR-mediated polyneuropathy (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 LUNARTM, 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 (UNATM) 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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