



Arcturus Therapeutics to Present Messenger RNA Pipeline at the 33rd Annual J.P. Morgan Healthcare Conference and the 7th Annual Biotech Showcase

January 7, 2015

Announces new messenger RNA therapeutics pipeline using LUNAR™ delivery technology and UNA-messenger RNA (UmrRNA)

Messenger RNA pipeline programs include thrombopoietin (LUNAR-TPO) for the management of thrombocytopenia, ornithine transcarbamylase (LUNAR-OTC) for the treatment of ornithine transcarbamylase deficiency, hepcidin (LUNAR-HPN) for the treatment of iron disorders, and cystic fibrosis transmembrane conductance regulator (LUNAR-CF) for the treatment of cystic fibrosis

San Diego, Calif., Jan 7, 2015 – Arcturus Therapeutics Inc., developing the ‘next wave’ of RNA medicines, today announced that Joseph Payne, President and Chief Executive Officer, is scheduled to present a corporate overview, including the company’s new messenger RNA (mRNA) therapeutics pipeline, at two upcoming healthcare conferences:

Event: 7th Annual Biotech Showcase

When: January 13, 2015 at 8:15 AM PST

Where: A-Hearst (4th Floor), Parc 55 Wyndham Union Square, San Francisco, CA

Event: 33rd Annual J.P. Morgan Healthcare Conference

When: January 14, 2015 at 5:00 PM PST

Where: Elizabethan D (private company track), Westin St. Francis Hotel, San Francisco, CA

“Since the inception of Arcturus we have been building our mRNA capabilities. We have proven LUNAR™ to be a potent and well tolerated delivery system for mRNAs and have demonstrated biological proof of concept for the up-regulation of erythropoietin and alpha-1 antitrypsin, using modified messenger RNA in non-human primates,” said Joseph Payne, President and Chief Executive Officer. “Further, we have built in-house mRNA synthesis capabilities at our new facility and have shown that the incorporation of UNA into mRNA (UmrRNA) can increase stability and therefore longevity to mRNA medicines. Both LUNAR™ and UNA technologies are wholly-owned by Arcturus providing the company with a unique position from which to capitalize on its leading RNA delivery and chemistry technologies for the burgeoning field of messenger RNA therapeutics.”

Messenger RNA provides a novel way for the body to naturally express therapeutic molecules to treat diseases that were previously inaccessible. The newly announced Arcturus pipeline of mRNA programs include:

LUNAR-TPO: LUNAR-formulated thrombopoietin (TPO) mRNA for the treatment of thrombocytopenia, which often occurs as a result of a separate disorder, such as HCV, leukemia, an immune system problem, or as a medication side effect in the management of patients with cancer. TPO is a hormone produced by the liver and kidney, and is the most potent cytokine that regulates platelet production. The recovery of platelets after bone marrow transplantation or chemotherapy often occurs later than the recovery of other hematopoietic lineages and thus patients may require platelet transfusion to prevent hemorrhage. Even though platelet transfusion reduces the risk of hemorrhage, about 30% of transfusions result in complications including transmission of viral disease, febrile reactions, alloimmunization and sepsis. LUNAR-TPO is an example of the Arcturus approach to developing mRNA therapeutics for secreted proteins.

LUNAR-OTC: LUNAR-formulated ornithine transcarbamylase (OTC) mRNA for the treatment of ornithine transcarbamylase deficiency, the most common inherited urea cycle disorder in humans. OTC is a liver enzyme responsible for detoxification of ammonia and, if a person is deficient in OTC, ammonia levels will build up in the blood resulting in neurological problems and other toxicities. One in 70,000 adults has an ornithine transcarbamylase deficiency although reports suggest late-onset cases may go undetected such that overall incidence may be in the range of 1:20,000. Clinical onset is often rapid and devastating in a patient who is genetically affected; however, in older individuals, the initial onset can occur at age 40-50 years or older. Diagnosis includes the measurement of ammonia levels in the serum, which provides a convenient biomarker for preclinical and clinical assessment of LUNAR-OTC. The only curative approach is liver transplantation. LUNAR-OTC is an example of the Arcturus approach to developing mRNA therapeutics for intracellular proteins.

LUNAR-HPN: LUNAR-formulated hepcidin mRNA for the treatment of iron disorders. Hepcidin is a hormone synthesized and secreted by the liver that serves as a key regulator of iron levels in the circulation. Iron balance must be carefully regulated to provide iron as needed while avoiding toxicity associated with excess. Excessive iron absorption and tissue iron overload is one of the main features of Beta-thalassemia. Because the main cause of increased iron absorption in Beta-thalassemia is the low expression of hepcidin levels, a novel treatment strategy overexpressing hepcidin to limit iron overload has significant commercial potential. LUNAR-HPN is an example of the Arcturus approach to developing mRNA therapeutics for the liver.

LUNAR-CF: LUNAR-formulated cystic fibrosis transmembrane conductance regulator (CFTR) mRNA for the treatment of cystic fibrosis (CF), the most frequent lethal genetic disease in the Caucasian population. One in 2,500 newborns in the Caucasian population are affected by CF. CFTR is the main chloride channel in secretory epithelia and also acts as a regulator of sodium transport. This imbalance of active ion transport favors the net removal of salt and water from airway surfaces causing dehydration of the airway surface liquid, thereby impeding mucociliary clearance and creating a favorable microenvironment for bacterial infections. LUNAR-CF is an example of the Arcturus approach to developing mRNA therapeutics for the lung.

About Arcturus Therapeutics, Inc.

Founded in 2013 and based in San Diego, Arcturus Therapeutics is developing the 'next wave' of RNA medicines. Arcturus has invented a novel, potent and safe RNA Therapeutics platform called LUNAR™, a proprietary lipid-enabled delivery system for RNA medicines including *BOTH* small interfering RNA (siRNA) and messenger RNA (mRNA) therapeutics. The company's UNA Oligomer™ chemistry technology and patent portfolio (34 patents, USPTO granted) enables the targeting of any gene in the human genome. Building a robust pipeline, Arcturus has focused internal efforts on RNA medicines for the treatment of rare diseases and, for larger indications, will be partnering with select companies that bring disease expertise. For more information, visit www.ArcturusRx.com.

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