Arcturus Therapeutics Reassumes Full Worldwide Rights to ARCT-810, a Clinical Development Candidate for Ornithine Transcarbamylase (OTC) Deficiency

February 11, 2019

SAN DIEGO, Feb. 11, 2019 (GLOBE NEWSWIRE) -- Arcturus Therapeutics Ltd. (NASDAQ: ARCT), a leading RNA medicines company focused on the discovery, development and commercialization of therapeutics towards rare diseases, today announced that it will reassume 100% global rights for its flagship asset, clinical development candidate ARCT-810, a messenger RNA (mRNA) drug to treat OTC Deficiency. ARCT-810 was previously subject to a 50/50 collaboration between Arcturus and CureVac AG. CureVac elected not to continue its obligations for the preclinical development of ARCT-810 under and pursuant to the terms of the collaboration.

The preclinical development program for ARCT-810, including Investigational New Drug Application (IND) enabling studies, remains on track. Arcturus is planning to file an IND for ARCT-810 with the U.S. Food and Drug Administration (FDA) in the fourth quarter of 2019.

CureVac remains committed to developing additional assets within the Arcturus collaboration utilizing CureVac mRNA and Arcturus LUNAR® delivery technology. CureVac and Arcturus will announce when the next program has been selected.

"We have had a very productive collaboration with CureVac on ARCT-810 and are pleased that we have secured all of the rights to this clinical development candidate. We are enthusiastic about the value of ARCT-810 and we believe it has the potential to be a transformational treatment for patients suffering from OTC deficiency," said Joseph Payne, President & CEO of Arcturus. "Importantly, Arcturus has the resources and expertise to advance this program into the clinic. We are pursuing an aggressive development timeline with an IND filing planned for this year. We look forward to our continued collaboration with CureVac to advance therapies for patients in need of potential new treatment options."

Arcturus plans to present further data and updates on the progress of ARCT-810 at the "TIDES: Oligonucleotide & Peptide Therapeutics" conference, to take place May 20th to 23rd, 2019 in San Diego, CA.

About ARCT-810
ARCT-810, Arcturus' first development candidate, represents a novel approach to treat ornithine transcarbamylase deficiency (OTCD). ARCT-810 is based on mRNA and also utilizes Arcturus' propriety lipid library and employs the Company's LUNAR ® delivery platform to safely and effectively deliver OTC mRNA to hepatocytes. ARCT-810 is an mRNA replacement therapy designed to enable OTC-deficient patients to naturally produce healthy functional OTC enzyme in their own liver cells. Arcturus is currently on track to submit an Investigational New Drug Application (IND) to the FDA in the fourth quarter of 2019. ARCT-810 is advancing toward the clinic on the strength of preclinical proof-of-concept data, demonstrating that LUNAR technology can deliver mRNA to liver cells and results in expression of functional OTC protein in animal models. Replacing the deficient OTC protein restores the urea cycle pathway, resulting in reduced plasma ammonia and urinary orotate concentrations.

About Ornithine Transcarbamylase Deficiency (OTCD)
OTC deficiency is caused by mutations in the OTC gene which leads to a non-functional or deficient OTC enzyme. OTCD is the most common urea cycle disorder. Urea cycle disorders are a group of inherited metabolic disorders that make it difficult for afflicted patients to remove toxic waste products, as proteins are digested. OTC deficiency is a life-threatening genetic disease. OTC is a critical enzyme in the urea cycle, which takes place in liver cells, and converts ammonia to urea. This conversion does not occur properly in patients with OTC deficiency and ammonia accumulates in their blood, acting as a neurotoxin and liver toxin. This can cause severe symptoms including vomiting, headaches, coma and death. OTC deficiency is an inherited disease that can cause developmental problems, seizures and death in newborn babies. It is an X-linked disorder, so is more common in boys. Patients with less severe symptoms may present later in life, as adults. There is currently no cure for OTC deficiency, apart from liver transplant. However, this treatment comes with significant risk of complications such as organ rejection, and transplant recipients must take immunosuppressant drugs for the rest of their lives. Current standard of care for OTC patients is a low-protein diet and ammonia scavengers to try and prevent patients from accumulating ammonia. These treatments do not address the underlying cause of disease.

About Arcturus Therapeutics Ltd.
Founded in 2013 and headquartered in San Diego, California, Arcturus Therapeutics Ltd. (NASDAQ: ARCT) is an RNA medicines company with enabling technologies – LUNAR® lipid-mediated delivery and UNA Oligomer chemistry. Arcturus' diverse pipeline of RNA therapeutics includes programs pursuing rare diseases, Hepatitis B, non-alcoholic steatohepatitis (NASH), cystic fibrosis, and vaccines. Arcturus' versatile RNA therapeutics platforms can be applied toward multiple types of nucleic acid medicines including messenger RNA, small interfering RNA, replicon RNA, antisense RNA, microRNA, DNA and gene editing therapeutics. Arcturus owns LUNAR lipid-mediated delivery and Unlocked Nucleomonomer Agent (UNA) technology including UNA Oligomers, which are covered by its extensive patent portfolio (150 patents and patent applications, issued in the U.S., Europe, Japan, China and other countries). Arcturus' proprietary UNA technology can be used to target individual genes in the human genome, as well as viral genes, and other species for therapeutic purposes. Arcturus' commitment to the development of novel RNA therapeutics has led to partnerships with Janssen Pharmaceuticals, Inc., part of the Janssen Pharmaceutical Companies of Johnson & Johnson, Ultragenyx Pharmaceutical, Inc., Takeda Pharmaceutical Company Limited, Synthetic Genomics Inc., CureVac AG and the Cystic Fibrosis Foundation. For more information, visit www.Arcturusrx.com, the content of which is not incorporated herein by reference.

Forward-Looking Statements
This press release contains "forward-looking statements" that involve substantial risks and uncertainties for purposes of the safe harbor provided by

the Private Securities Litigation Reform Act of 1995. Any statements, other than statements of historical fact, included in this press release regarding strategy, future operations, collaborations, future financial position, prospects, plans and objectives of management, including statements relating to the status of the preclinical development program for ARCT-810, the date that an IND may be filed with the FDA, the potential for ARCT-810, are forward-looking statements. Arcturus may not actually achieve the plans, carry out the intentions or meet the expectations or projections disclosed in any forward-looking statements and you should not place undue reliance on such forward-looking statements. Actual results and performance could differ materially from those projected in any forward-looking statements as a result of many factors, including without limitation, an inability to develop and market product candidates. Such statements are based on management's current expectations and involve risks and uncertainties, including those discussed under the heading "Risk Factors" in Arcturus' Annual Report on Form 20-F for the fiscal year ended December 31, 2017, filed with the SEC on May 14, 2018 and in subsequent filings with, or submissions to, the SEC. Except as otherwise required by law, Arcturus disclaims any intention or obligation to update or revise any forward-looking statements, which speak only as of the date they were made, whether as a result of new information, future events or circumstances or otherwise.

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Source: Arcturus Therapeutics, Inc.