Arcturus Therapeutics to Develop ARCT-810 for Treatment of Ornithine Transcarbamylase (OTC) Deficiency

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SAN DIEGO, Jan. 02, 2019 (GLOBE NEWSWIRE) -- Arcturus Therapeutics Ltd. (NASDAQ: ARCT), a leading RNA medicines company focused on the development and commercialization of therapeutics towards rare, liver and respiratory diseases with significant unmet medical need, today announced the nomination of its first development candidate, ARCT-810, to treat ornithine transcarbamylase deficiency (OTCD). 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. ARCT-810 is based on Arcturus's propriety lipid library which utilizes the company's LUNAR® delivery platform to safely and effectively deliver OTC messenger RNA to liver cells.

"We are pleased to select ARCT-810 as a candidate for clinical development," said Joseph Payne, President and Chief Executive Officer of Arcturus Therapeutics. "OTC deficiency is a life-threatening genetic disease. With our collaboration partner, CureVac, we are working to develop a messenger RNA replacement therapy that's designed to enable OTC-deficient patients to naturally produce healthy functional OTC enzyme in their own liver cells. We are currently on track to submit an Investigational New Drug Application (IND) to the FDA in the second half of 2019."

"Our development candidate, ARCT-810, represents a novel approach to treat OTC deficiency," said Dr. Pad Chivukula, Chief Scientific Officer of Arcturus. "We are moving into the clinic on the strength of preclinical proof-of-concept in our LUNAR-OTC program, demonstrating that LUNAR technology can deliver messenger RNA 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. We look forward to IND-enabling studies which will allow the advancing of ARCT-810 into the clinic and investigation of its utility in humans."

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. 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 based in San Diego, California, Arcturus Therapeutics Ltd. (NASDAQ: ARCT) is an RNA medicines company with enabling technologies – UNA Oligomer chemistry and LUNAR® lipid-mediated delivery. 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 RNA medicines including small interfering RNA, messenger RNA, replicon RNA, antisense RNA, microRNA 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 (140 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 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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