

# Arcturus Therapeutics Receives up to \$15 Million Commitment from the Cystic Fibrosis Foundation to Create mRNA Therapies to Treat Cystic Fibrosis Patients

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SAN DIEGO, Aug. 01, 2019 (GLOBE NEWSWIRE) -- Arcturus Therapeutics ("Arcturus" or the "Company"), a leading RNA medicines company, today announced that the Cystic Fibrosis Foundation (CF Foundation), has increased its commitment to \$15 million in conjunction with an amended agreement to advance LUNAR-CF, a novel messenger RNA (mRNA) therapeutic formulated with Arcturus' LUNAR® delivery technology.

The goal of the multi-year program is to create mRNA therapies to treat people with cystic fibrosis (CF), develop methods to deliver RNA components to cells in the lung and file an Investigational New Drug (IND) application for a therapeutic candidate.

"We are pleased with the progress we have made in our agreement with the CF Foundation, including preclinical proof of concept studies, demonstrating that LUNAR is able to deliver mRNA efficiently into lung epithelial cells in animals and is compatible with nebulization," said Joseph Payne, President and CEO of Arcturus Therapeutics. "We are delighted with the expanded financial support from the CF Foundation, and we believe that we are now sufficiently funded to advance the LUNAR-CF program into filing an IND application."

Arcturus has collaborated with the CF Foundation since 2017.

#### **About LUNAR-CF**

LUNAR-CF, Arcturus' first inhaled mRNA therapeutic targeting the lung, represents a novel approach to treat cystic fibrosis (CF). LUNAR-CF is based on Arcturus messenger RNA (mRNA) design construct and proprietary manufacturing process. LUNAR-CF also utilizes Arcturus' propriety lipid library and employs the Company's LUNAR<sup>®</sup> delivery platform to safely and effectively deliver CFTR mRNA to the lung. LUNAR-CF is an mRNA replacement therapy designed to enable CFTR-deficient patients to naturally produce healthy functional CFTR in their own lung cells. Arcturus plans to submit an Investigational New Drug (IND) application to the FDA in the second half of 2020. LUNAR-CF is advancing toward the clinic on the strength of preclinical proof-of-concept data, demonstrating that LUNAR technology can deliver mRNA to bronchial epithelial cells and results in expression of CFTR protein in animal models.

### **About Arcturus Therapeutics**

Founded in 2013 and based in San Diego, California, Arcturus Therapeutics Holdings Inc. (NASDAQ: ARCT) is an RNA medicines company with enabling technologies – LUNAR® lipid-mediated delivery and Unlocked Nucleomonomer Analog (UNA) chemistry – and mRNA drug substance along with drug product manufacturing. Arcturus' diverse pipeline of RNA therapeutics includes programs to potentially treat Ornithine Transcarbamylase (OTC) Deficiency, Cystic Fibrosis, Glycogen Storage Disease Type 3, Hepatitis B, and non-alcoholic steatohepatitis (NASH). 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 technologies are covered by its extensive patent portfolio (167 patents and patent applications, issued in the U.S., Europe, Japan, China and other countries). Arcturus' commitment to the development of novel RNA therapeutics has led to collaborations with Janssen Pharmaceuticals, Inc., part of the Janssen Pharmaceutical Companies of Johnson & Johnson, Ultragenyx Pharmaceutical, Inc., Takeda Pharmaceutical Company Limited, CureVac AG, Synthetic Genomics Inc. and the Cystic Fibrosis Foundation. For more information, visit <a href="https://www.ArcturusRx.com">www.ArcturusRx.com</a>.

## **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, the likelihood of success of the Company's technology or potential development of any products, the status of the preclinical development program for any of the clinical development programs of Arcturus, the status of IND-enabling studies and early clinical development related to any of the clinical development programs of Arcturus, the sufficiency of any drug substances or drug products of the Company to meet the Company's current clinical goals or expectations, the date that an IND may be filed with the FDA, the Company's ability or expectation to file an IND for any of its product candidates, the potential market or success for the clinical development programs of Arcturus, current standards of care, and the Company's future cash and financial position 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 such as the foregoing 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, inability to generate positive verifiable data, unexpected clinical results, unforeseen expenses and general market conditions that may prevent such achievement or performance. 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 10-K for the fiscal year ended December 31, 2018, filed with the SEC on March 18, 2019 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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