



Arcturus Therapeutics to Advance ARCT-032, an Aerosolized LUNAR® mRNA-based Therapeutic, as a Development Candidate for Cystic Fibrosis Lung Disease

December 30, 2020

SAN DIEGO--(BUSINESS WIRE)--Dec. 30, 2020-- Arcturus Therapeutics Holdings Inc. (the "Company", "Arcturus", Nasdaq: ARCT), a leading clinical-stage messenger RNA medicines company focused on the development of infectious disease vaccines and significant opportunities within liver and respiratory rare diseases, today announced that it has selected ARCT-032, an aerosolized LUNAR® mRNA-based therapeutic candidate, as a development candidate for Cystic Fibrosis (CF), a progressive hereditary disease.

"We are pleased to have advanced ARCT-032 as a novel mRNA-based development candidate for CF Lung Disease. ARCT-032, based on our proprietary LUNAR® technology, is designed to result in the efficient expression of a functional Cystic Fibrosis Transmembrane Conductance Regulator (CFTR) protein in the lungs. We believe that ARCT-032 has the potential to address the root cause of CF lung disease, which is caused by the defective, or missing, CFTR protein," said Pad Chivukula, Ph.D. Chief Scientific Officer and Chief Operating Officer of Arcturus Therapeutics. "While there has been progress with the recent approval of drugs for CF, many patients remain underserved. ARCT-032 has the potential to provide benefit to all CF patients, regardless of their underlying genetic mutations."

About ARCT-032

ARCT-032 will utilize Arcturus' LUNAR® lipid-mediated aerosolized platform to deliver CFTR messenger RNA to the lungs. Expression of a functional copy of the CFTR mRNA in the lungs of CF patients has the potential to restore normal lung CFTR activity. The ARCT-032 program is supported by preclinical data in CFTR deficient murine model, ferrets and NHPs. LUNAR®-CFTR can be efficiently delivered to epithelial cells in the airways and restore chloride channel activity in a CFTR KO mice model following intranasal administration of LUNAR®-CFTR.

An mRNA-based replacement therapy for CF, if successfully developed, has the potential to deliver a fully functional copy of CFTR into the lungs, independent of the underlying CF genotype. The expression of functional CFTR protein is expected to restore chloride channel efflux in the airways, reducing mucus accumulation, tissue scarring, and minimizing the progressive respiratory dysfunction observed in CF patients.

About Arcturus Therapeutics

Founded in 2013 and based in San Diego, California, Arcturus Therapeutics Holdings Inc. (Nasdaq: ARCT) is a clinical-stage mRNA medicines and vaccines company with enabling technologies: (i) LUNAR® lipid-mediated delivery, (ii) STARR™ mRNA Technology and (iii) mRNA drug substance along with drug product manufacturing expertise. Arcturus' diverse pipeline of RNA therapeutic and vaccine candidates includes self-replicating mRNA vaccine programs for SARS-CoV-2 (COVID-19) and Influenza, and other programs to potentially treat Ornithine Transcarbamylase (OTC) Deficiency, Cystic Fibrosis, and Cardiovascular Disease along with partnered programs including Glycogen Storage Disease Type 3, Hepatitis B Virus, 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 (200 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., Duke-NUS Medical School, and the Cystic Fibrosis Foundation. For more information visit www.ArcturusRx.com. In addition, please connect with us on [Twitter](https://twitter.com/ArcturusRx) and [LinkedIn](https://www.linkedin.com/company/arcturus-therapeutics).

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, are forward-looking statements, including those regarding the Company's development strategy, the likelihood that preclinical or clinical results will be predictive of future clinical results or sufficient for regulatory approval, the likelihood of the Company to obtain clearance from regulatory authorities to proceed with planned clinical trials, the planned initiation, design or completion of clinical trials, the likelihood of success, or of the efficacy or safety, of ARCT-032 or other product candidates, future operations, the continuation or success of collaborations with the Company's strategic partners, and our current cash position and expected cash burn. 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, the impact of COVID-19 on the ability to conduct clinical trials, the availability of manufacturing capacity and raw materials, unexpected clinical results, and general market conditions that may prevent such achievements 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, 2019, filed with the SEC on March 16, 2020 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.

IR and Media Contacts

Arcturus Therapeutics
Neda Safarzadeh
(858) 900-2682
IR@ArcturusRx.com

Kendall Investor Relations
Carlo Tanzi, Ph.D.
(617) 914-0008
ctanzi@kendallir.com

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