Arcturus Therapeutics Announces Clinical Trial Application for ARCT-032 Received Approval to Proceed into First-in-Human Studies to Treat Cystic Fibrosis

January 31, 2023

SAN DIEGO--(BUSINESS WIRE)--Jan. 31, 2023-- Arcturus Therapeutics Holdings Inc. (the “Company”, “Arcturus”, “Arcturus Therapeutics”, Nasdaq: ARCT), a global late-stage clinical messenger RNA medicines company focused on the development of infectious disease vaccines and significant opportunities within liver and respiratory rare diseases, today announced that the Clinical Trial Application (CTA) for ARCT-032, an inhaled investigational mRNA medicine to treat cystic fibrosis (CF), received approval to proceed into a Phase 1 First-in-Human study in New Zealand.

“Arcturus continues to progress ARCT-032, a potential mRNA medicine for people with cystic fibrosis regardless of their underlying mutation type, through the approval of a CTA to proceed into First-in-Human studies,” said Joseph Payne, President and CEO of Arcturus Therapeutics. “We believe the advantages driven by our proprietary LUNAR® delivery technology, advanced mRNA purification processes, and manufacturing know-how may allow ARCT-032 to restore healthy CFTR protein in the lungs of people with CF, including those that currently do not have effective treatment options. Preclinical data shared at the recent North American Cystic Fibrosis Conference demonstrated robust expression and functional restoration of CFTR in human bronchial epithelial cells from CF donors, providing additional support for the advancement of ARCT-032 into clinical development.”

About Cystic Fibrosis

Cystic fibrosis is a life-shortening disease with a worldwide distribution. Mutations in the Cystic Fibrosis Transmembrane (CFTR) gene result in a reduction or absence of CFTR quantity and/or function in the airways, causing insufficient chloride transport to maintain airway surface homeostasis. CF mucus is more difficult to clear, thus clogging the airways and leading to infection, inflammation, respiratory failure, or other life-threatening complications. Currently approved CFTR modulator therapies are designed to increase function of the CFTR channel to help reduce symptoms yet are ineffective in many people with CF as a result of their underlying 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 people with CF has the potential to restore CFTR activity and mitigate the downstream effects that cause progressive lung disease. The ARCT-032 program is supported by preclinical data in rodents, ferrets and primates, as well as demonstrating restoration of expression and function in human bronchial epithelial cells.

About Arcturus Therapeutics

Founded in 2013 and based in San Diego, California, Arcturus Therapeutics Holdings Inc. (Nasdaq: ARCT) is a global late-stage clinical mRNA medicines and vaccines company with enabling technologies: (i) LUNAR® lipid-mediated delivery, (ii) STARR™ mRNA Technology (samRNA) and (iii) mRNA drug substance along with drug product manufacturing expertise. Arcturus’ diverse pipeline of RNA therapeutic and vaccine candidates includes mRNA vaccine programs for SARS-CoV-2 (COVID-19) and Influenza, and other programs to potentially treat ornithine transcarbamylase (OTC) deficiency, and cystic fibrosis, along with partnered programs including glycogen storage disease type III, and hepatitis B virus. Arcturus’ versatile RNA therapeutics platforms can be applied toward multiple types of nucleic acid medicines including messenger RNA, small interfering RNA, circular RNA, antisense RNA, self-amplifying RNA, DNA, and gene editing therapeutics. Arcturus’ technologies are covered by its extensive patent portfolio (patents and patent applications issued in the U.S., Europe, Japan, China and other countries). For more information, visit www.ArcturusRx.com. In addition, please connect with us on Twitter and LinkedIn.

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 strategy, future operations, the expectations for or likelihood of success of any collaborations, the likelihood of success (including safety and efficacy) of ARCT-032, the planned initiation, design or completion of clinical trials (including the planned ARCT-032 Phase 1 study), the likelihood that preclinical data will be predictive of future clinical results, the likelihood that ARCT-032 will provide broad, or any, clinical benefit (including the ability of ARCT-032 to restore healthy CFTR protein), the ability to enroll, and timing for enrollment of, subjects in clinical trials, the timing and nature of any study results, the likelihood that a patent will issue from any patent application, and the impact of general business and economic conditions. 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. These statements are only current predictions or expectations, and are subject to known and unknown risks, uncertainties, and other factors that may cause our or our industry’s actual results, levels of activity, performance or achievements to be materially different from those anticipated by the forward-looking statements, including those discussed under the heading "Risk Factors" in Arcturus’ most recent Annual Report on Form 10-K, and in subsequent filings with, or submissions to, the SEC, which are available on the SEC’s website at www.sec.gov. 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.

View source version on businesswire.com: https://www.businesswire.com/news/home/20230131005546/en/

IR and Media Contacts
Arcturus Therapeutics
Neda Safarzadeh
VP, Head of IR/PR/Marketing
(858) 900-2682
IR@ArcturusRx.com

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

Source: Arcturus Therapeutics Holdings Inc.