Arcturus Therapeutics Receives Clearance of an Investigational New Drug Application to U.S. Food and Drug Administration for ARCT-032, an Investigational Inhaled mRNA Therapeutic to Treat Cystic Fibrosis

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SAN DIEGO--(BUSINESS WIRE)--Sep. 3, 2024-- Arcturus Therapeutics Holdings Inc. (the “Company”, “Arcturus”, Nasdaq: ARCT), a global messenger RNA medicines company focused on the development of infectious disease vaccines and addressing unmet medical needs within liver and respiratory rare diseases, today announced that the U.S. Food and Drug Administration (FDA) has issued a “Study May Proceed” notification for the Company’s Investigational New Drug (IND) application, ARCT-032, to treat cystic fibrosis (CF). FDA clearance of the ARCT-032 IND application enables the Company to initiate a Phase 2 multiple ascending dose study to evaluate the safety, tolerability and efficacy of ARCT-032 in people with Cystic Fibrosis.

“The Phase 2 Study May Proceed notification allows us to investigate ARCT-032 as a potential treatment for CF patients and provides the opportunity to further validate our LUNAR® technology to deliver mRNA via inhalation,” said Dr. Juergen Froehlich, Chief Medical Officer of Arcturus Therapeutics. “The study is designed to evaluate the safety and effectiveness of ARCT-032 administered for several weeks at multiple dose levels in people with CF who do not qualify for, or benefit from, CFTR modulator therapy.”

About Cystic Fibrosis

Cystic fibrosis is a life-shortening disease with a worldwide prevalence. Mutations in the cystic fibrosis transmembrane conductance regulator (CFTR) gene result in a reduction or absence of CFTR protein 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. Standard of care for many CF individuals include CFTR modulators. Nearly 40,000 people in the U.S. and more than 105,000 people worldwide are living with CF. Approximately 15% of individuals with CF do not benefit from CFTR modulator medicines due to dysfunctional or absent CFTR protein and/or drug intolerance.

About ARCT-032

ARCT-032 is an inhaled investigational mRNA therapeutic designed to express normal functional CFTR in the lungs of individuals with CF. ARCT-032 has received Orphan Medicinal Product Designation from the European Medicines Agency (EMA) and Orphan Drug Designation along with Rare Pediatric Disease Designation from the U.S. Food and Drug Administration (FDA) to treat cystic fibrosis. ARCT-032 utilizes Arcturus' LUNAR® lipid-mediated aerosolized platform to deliver CFTR messenger RNA to the lungs. Lung disease is the leading cause of morbidity and mortality in people with CF. 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 CFTR 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 mRNA medicines and vaccines company with enabling technologies: (i) LUNAR® lipid-mediated delivery, (ii) STARR® mRNA Technology (sa-mRNA) and (iii) mRNA drug substance along with drug product manufacturing expertise. Arcturus developed KOSTAIVE®, the first self-amplifying messenger RNA (sa-mRNA) COVID vaccine in the world to be approved. Arcturus has an ongoing global collaboration for innovative mRNA vaccines with CSL Seqirus, and a joint venture in Japan, ARCALIS, focused on the manufacture of mRNA vaccines and therapeutics. Arcturus’ pipeline includes RNA therapeutic candidates to potentially treat ornithine transcarbamylase (OTC) deficiency and cystic fibrosis (CF), along with its partnered mRNA vaccine programs for SARS-CoV-2 (COVID-19) and influenza. 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 (over 400 patents and patent applications 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 likelihood of success and continued advancement of the Company’s pipeline and partnered programs, and the potential initiation of a Phase 2 study of ARCT-032 and the timing thereof. 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

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