Arcturus Therapeutics Receives Orphan Medicinal Product Designation from the European Commission (EC), for ARCT-032, for the Treatment of Cystic Fibrosis

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Access to protocol assistance, centralized authorization process, fee reductions and 10 years of market exclusivity

SAN DIEGO--(BUSINESS WIRE)--Feb. 22, 2024-- Arcturus Therapeutics Holdings Inc. (the “Company”, “Arcturus”, Nasdaq: ARCT), a global late-stage clinical messenger RNA medicines company focused on the development of infectious disease vaccines and medicines to treat unmet medical needs within liver and respiratory rare diseases, today announced that the European Commission (EC), based on a positive opinion issued by the European Medicines Agency (EMA), has granted orphan medicinal product designation for the Company’s product candidate ARCT-032 to treat Cystic Fibrosis (CF). In November 2023, the U.S. Food and Drug Administration (FDA) granted orphan drug designation to ARCT-032 for CF.

Orphan medicinal product designation by the EC is available to novel therapeutics that prevent or treat life-threatening or chronically debilitating conditions that affect fewer than five in 10,000 persons in the EU. The designation provides significant incentives to promote the development of the drug including protocol assistance, access to the centralized authorization procedure, fee reductions, and research grants, as well as 10 years of market exclusivity.

“We are pleased to receive orphan medicinal product designation from the EC for ARCT-032, as it represents a significant milestone for our CF program,” said Joseph Payne, President & CEO of Arcturus Therapeutics. “We will continue to advance ARCT-032 as a potential new treatment option for people with CF regardless of their genetic mutations.”

After successful completion of the single-ascending-dose portion of the study in healthy adults, the first participant with CF in the Phase 1b study part enrolled and completed two administrations of ARCT-032. Arcturus remains on track to share interim Phase 1b data in H1 2024.

About Cystic Fibrosis

Cystic fibrosis is a life-shortening disease with a worldwide distribution. 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. Currently approved CFTR modulator therapies are designed to increase function of the CFTR channel to help reduce symptoms yet are ineffective in some people with CF because of their underlying mutations.

About ARCT-032

ARCT-032 utilizes 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 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 late-stage clinical 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 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 deficiency and cystic fibrosis, 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 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 the continued clinical development of ARCT-032, the likelihood of sharing and timing for sharing interim Phase 1b data, likelihood of success (including safety and efficacy) of ARCT-032, the Company’s strategy and future operations, 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.

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