Arcturus Therapeutics Receives U.S. FDA Fast Track Designation for ARCT-810, mRNA Therapeutic Candidate for Ornithine Transcarbamylase Deficiency

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SAN DIEGO--(BUSINESS WIRE)--Jun. 1, 2023-- 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 opportunities within liver and respiratory rare diseases, today announced that the U.S. Food and Drug Administration (FDA) has granted Fast Track Designation to ARCT-810, the Company’s mRNA therapeutic candidate for ornithine transcarbamylase (OTC) deficiency.

Fast Track Designation is designed to facilitate development and expedite review of new therapeutics intended to treat serious or life-threatening conditions that demonstrate the potential to address important unmet medical needs. Programs granted Fast Track Designation may receive important benefits including more frequent interactions with FDA review teams and the ability to obtain rolling review of a Biologics License Application (BLA). In addition, BLA applications may receive a priority review, with the FDA aiming to complete its review within six months, instead of the standard ten months review timeline.

“ARCT-810 has the potential to be an important new medicine for individuals living with OTC deficiency and we are very pleased to have obtained Fast Track Designation for this program. This FDA designation provides several meaningful benefits that we expect will accelerate development and, hopefully, support the approval of this investigational therapy,” said Juergen Froehlich, MD, MBA, FCP, Chief Medical Officer, of Arcturus Therapeutics. “We look forward to more frequent interactions with the FDA and to acceleration of ARCT 810 as a potential new treatment option that addresses the root cause of OTC deficiency.”

About Ornithine Transcarbamylase (OTC) Deficiency

OTC deficiency is a serious urea cycle disorder with a prevalence of approximately 10,000 people worldwide. A lack of OTC, a critical urea cycle enzyme in liver cells, results in high blood ammonia levels that can cause diminished cognitive ability, seizures, coma and death. The current standard of care for OTC-deficient patients is a low-protein diet, nutritional supplements and ammonia scavengers to help prevent the accumulation of ammonia. These treatments do not address the underlying cause of OTC deficiency, for which there are no FDA approved medicines.

About ARCT-810

ARCT-810 is a development candidate that represents a novel approach to treat ornithine transcarbamylase deficiency. ARCT-810 utilizes Arcturus’ mRNA design construct and proprietary manufacturing process. ARCT-810 was developed using Arcturus’ extensive and proprietary lipid library and employs its LUNAR® platform to deliver OTC mRNA to hepatocytes. ARCT-810 is an investigational mRNA medicine designed to enable OTC-deficient patients to naturally produce functional OTC enzyme in their own liver cells. Enabling patients to produce their own OTC enzyme has the potential to restore urea cycle activity, reduce the risk of metabolic decompensation, and improve quality of life.

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’ 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 (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 likelihood of success of the Company’s pipeline (including ARCT-810) and partnered programs, the potential for the Company’s platform to result in novel vaccines or therapeutics, the benefits that the Company might receive from Fast Track Designation for ARCT-810, continued or accelerated development of ARCT-810, likelihood of approval of ARCT-810 or its potential for therapeutic benefit including restoration of urea cycle activity, 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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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

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