Arcturus Therapeutics Announces Completion of First Three Dose Escalation Cohorts in Phase 1 Study of ARCT-810, Therapeutic Candidate for Ornithine Transcarbamylase (OTC) Deficiency

October 5, 2020

LUNAR® lipids undetectable in plasma 48 hours following drug administration

ARCT-810 well tolerated at doses up to 0.3 mg/kg, 0.4 mg/kg cohort to be completed in Q4

Initial dosing of OTC-deficient patients at a U.S. clinical site expected in Q4

SAN DIEGO--(BUSINESS WIRE)--Oct. 5, 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 the completion of the first three dose escalation cohorts in its ongoing Phase 1 study with ARCT-810, the Company’s messenger RNA (mRNA)-based therapeutic candidate for Ornithine Transcarbamylase (OTC) deficiency.

The ARCT-810 Phase 1 study is a double blind, placebo-controlled, dose-escalation trial in healthy adult volunteers. The study has completed three cohorts in total and the fourth cohort is expected to complete this quarter. All cohorts are randomized 2:1 active to placebo and the study is designed to evaluate safety and tolerability, and pharmacokinetics, as primary and secondary endpoints.

All subjects in cohorts up to 0.3 mg/kg have completed dosing and all study visits. Based on the available preliminary study data, ARCT-810 has been well tolerated at these doses, which are all within the anticipated therapeutic range. All adverse events observed have been mild or moderate in nature and there have been no serious adverse events. ARCT-810 has demonstrated a favorable pharmacokinetic profile, and no ARCT-810 lipid was detectable in plasma beyond 48 hours following drug administration. The company plans to report final data following study completion, which is anticipated this quarter.

“We are pleased to have made rapid progress advancing our Phase 1 study for ARCT-810, a highly promising mRNA-based therapeutic candidate for OTC deficiency. Preliminary safety and pharmacokinetic data are favorable and supportive of continued development,” said Steve Hughes, M.D., Chief Development Officer of Arcturus. “Administration at the highest dose cohort is ongoing and we anticipate study completion later this quarter. Our second ARCT-810 clinical study in patients with OTC deficiency is now recruiting and we expect to begin dosing patients at a U.S. clinical site this quarter.”

About ARCT-810

ARCT-810 utilizes Arcturus' LUNAR® lipid-mediated delivery platform to deliver OTC messenger RNA to liver cells. Expression of ornithine transcarbamylase enzyme in the liver of patients with OTC deficiency has the potential to restore normal urea cycle activity, preventing neurological damage and the need for liver transplantation. The ARCT-810 program is supported by preclinical data in OTC deficiency murine models demonstrating that dosing of LUNAR-OTC results in robust ornithine transcarbamylase protein expression and activity resulting in improvements in ureagenesis and plasma ammonia, and increased survival.

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, 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 (198 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, and the Cystic Fibrosis Foundation. 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, including those regarding the Company’s expected performance, the Company’s development of any specific mRNA therapeutics or vaccines, the likelihood of success, efficacy or safety of ARCT-810, the likelihood that ARCT-810 preclinical data or existing clinical data will be predictive of future clinical data, the likelihood that available preliminary study data will be predictive of the full data sets when available, the expected dose size of ARCT-810, the timing or success of, or ability to initiate or complete, the current Phase 1 study for ARCT-810 or future clinical studies of ARCT-810, are forward-looking statements. 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. 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.

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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

Source: Arcturus Therapeutics Holdings Inc.