Arcturus Therapeutics Announces First Healthy Volunteer Dosed in Phase 1 Study of ARCT-810 for Ornithine Transcarbamylase (OTC) Deficiency

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SAN DIEGO, June 05, 2020 (GLOBE NEWSWIRE) -- Arcturus Therapeutics Holdings Inc. (the “Company”, “Arcturus”, Nasdaq: ARCT), a leading clinical-stage messenger RNA medicines company focused on the discovery, development and commercialization of therapeutics for rare diseases and vaccines, today announced that it has dosed the first healthy volunteer in a Phase 1 study with ARCT-810, the Company’s messenger RNA (mRNA)-based therapeutic candidate for Ornithine Transcarbamylase (OTC) deficiency.

“We are excited to advance ARCT-810, a highly promising mRNA-based therapeutic candidate for OTC deficiency, into a Phase 1 study. This important milestone marks Arcturus’ transition into a clinical stage biopharmaceutical company,” said Steve Hughes, M.D., Chief Development Officer of Arcturus. “ARCT-810 is supported by compelling preclinical data that provide support for this candidate’s potential to transform the lives of patients with OTC deficiency, a life-threatening disease.”

ARCT-810 utilizes Arcturus' LUNAR® lipid-mediated delivery platform to effectively 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.

Worldwide development and commercialization rights to ARCT-810 are entirely held by Arcturus.

The ARCT-810 Phase 1 study is a double blind, placebo-controlled, dose-escalation trial in healthy adult volunteers. The study will evaluate five cohorts, each with six subjects. In each cohort, four subjects receive ARCT-810 and two receive placebo. The study will evaluate safety and tolerability, pharmacokinetics, and pharmacodynamic biomarkers.

About Ornithine Transcarbamylase (OTC) deficiency
Ornithine Transcarbamylase (OTC) deficiency is a serious, urea cycle disorder with a prevalence of approximately 10,000 worldwide. A lack of the OTC enzyme in liver cells results in high blood ammonia levels and can cause seizures, coma, and death in untreated patients. There are no FDA approved medicines for OTC deficiency.

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 candidates includes programs to potentially treat Ornithine Transcarbamylase (OTC) Deficiency, Cystic Fibrosis, Glycogen Storage Disease Type 3, Hepatitis B, non-alcoholic steatohepatitis (NASH) and a self-replicating mRNA vaccine for SARS-CoV-2. 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 (187 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, Catalent Inc., and the Cystic Fibrosis Foundation. For more information visit www.ArcturusRx.com

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 novel mRNA therapeutics, the likelihood of success, efficacy or safety of ARCT-810, the ability to initiate or complete clinical development programs, the likelihood that preclinical data will be predictive of clinical data, the sufficiency of any drug substances or drug products of the Company to meet the Company’s current clinical goals or expectations and expectations of market exclusivity concerning clinical development programs 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. No assurances can be given that any results reported in pre-clinical studies can be replicated in further studies or in human beings, or that a vaccine can or will ever be developed or approved using the Company’s technology. 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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