Arcturus Therapeutics to Host Key Opinion Leader (KOL) Presentation of ARCT-810 Phase 2 Interim Data for Ornithine Transcarbamylase (OTC) Deficiency

June 23, 2025

SAN DIEGO--(BUSINESS WIRE)--Jun. 23, 2025-- Arcturus Therapeutics Holdings Inc. (the “Company”, “Arcturus”, Nasdaq: ARCT), a commercial messenger RNA medicines company focused on the development of infectious disease vaccines and opportunities within liver and respiratory rare diseases, today announced that it will host a virtual KOL presentation of ARCT-810 Phase 2 interim data for Ornithine Transcarbamylase (OTC) deficiency on Monday, June 30, 2025, at 12:00 p.m. ET. The Company will issue a press release summarizing the data at 11 am ET, prior to the presentation.

The KOL presentation will review Arcturus’ interim clinical data from ARCT-810 Phase 2 trials evaluating the safety and pharmacodynamics of multiple doses of ARCT-810 in adolescent and adult participants with OTC deficiency. In addition to the management team, the following KOLs will be participating in the presentation:

Marshall Summar, MD, CEO at Uncommon Cures®

- Previously Chief of the Division of Genetics and Metabolism, Director of the Rare Disease Institute and the Margaret O’Malley Chair of Genetic Medicine at Children’s National Hospital, Emeritus Professor of Pediatrics at The George Washington University. Dr. Summar is an internationally recognized expert in urea cycle disorders (UCDs) with over four decades of clinical, research, and policy leadership. He is a founding member and Executive Committee member of the NIH UCD Consortium, where he led national UCD diagnostic and treatment consensus efforts. For more than 20 years, he served on the Scientific Advisory Board of the National UCD Foundation and has advised numerous academic and industry initiatives. He is the author of over 40 UCD-related publications, including GeneReviews®, clinical guidelines, and multinational natural history studies. As an inventor, he holds patents for ammonia diagnostics and UCD-related technologies and has led translational research in critical care and neonatal disease. In recognition of his long-standing contributions to rare disease research and clinical infrastructure, he received the NORD Lifetime Achievement Award in 2022.

Johannes Häberle, MD, Professor and Director, Head of Metabolic Laboratory, Division of Metabolism, Urea Cycle Disorders Translational Center at the University of Zurich

- Globally recognized expert in pediatric metabolic medicine and inborn errors of metabolism, serving as head of the Metabolic Laboratory at the University Children’s Hospital Zürich and Adjunct Professor of Pediatrics at the University of Zürich. With over 200 publications and 10,000+ citations, his research focuses on urea cycle disorders (UCDs) and novel therapies, metabolic flux analysis, and humanized models. He chaired the European working group that developed and updated the 2019 UCD diagnosis and treatment guidelines, and holds major leadership roles, including until Spring 2025 Chair of the Education & Training Advisory Committee (ETAC) of the Society for the Study of Inborn Errors of Metabolism (SSIEM) and council member of SSIEM, and scientific board member of the European Registry and Network for Intoxication-Type Metabolic Diseases (E-IMD). He is a core member of ClinGen’s UCD Variant Curation Expert Panel and received the prestigious Archibald Garrod Award in 2019. He was appointed to the Scientific Supervisory Board of the Citrin Foundation in 2021. His work is supported by the Swiss National Science Foundation and the University of Zurich’s URPP ITINERARE program, and he currently leads translational research efforts through a newly founded UCD research center in Zürich.

Virtual KOL Presentation: Monday, June 30, 2025 @ 12:00 p.m. ET

- Domestic: 1-877-407-0784
- International: 1-201-689-8560
- Conference ID: Arcturus
- Webcast: Link

About Ornithine Transcarbamylase Deficiency

Ornithine transcarbamylase (OTC) deficiency is the most common urea cycle disorder. Urea cycle disorders are a group of inherited metabolic disorders of the liver that make it difficult for affected patients to remove toxic waste products as proteins are digested. OTC deficiency caused by mutations in the X-linked OTC gene, leads to a non-functional or deficient OTC enzyme and usually affects males more severely. OTC is a critical liver

enzyme which catalyzes a metabolic process that converts toxic ammonia to urea that is excreted by the kidney. This conversion does not occur properly in patients with OTC deficiency and, aside from the risk of high ammonia levels, leads to increased blood concentrations of glutamine with low to normal levels of citrulline and increases in urine orotic acid. High blood ammonia levels in OTC deficiency may cause health crises with seizures, progressive neurocognitive impairment, coma, and death. Severe cases of OTC deficiency usually present early in life, but patients with less severe symptoms may be diagnosed as adolescents and adults. There is currently no cure for OTC deficiency, apart from liver transplant. However, liver transplantation comes with significant risks of surgical and postsurgical complications such as organ rejection, and recipients must take immunosuppressant drugs for the rest of their lives. The current standard of care for OTC deficiency patients is a well-controlled, but challenging to maintain, low-protein diet, substitution of essential amino acids and treatment with nitrogen scavenging medications that keeps the ammonia from rising to acutely toxic levels but may not prevent chronic neurotoxic effects. These treatments do not address the underlying cause of disease.
In Europe and the U.S., approximately 10,000 people have OTC deficiency.

About ARCT-810

ARCT-810 is an intravenously administered investigational mRNA therapeutic designed to express normal functional OTC enzyme in the liver of individuals with OTC deficiency. ARCT-810 has received Orphan Medicinal Product Designation and an approved pediatric investigation plan (PIP) from the European Medicines Agency (EMA), and Orphan Drug Designation, Fast Track Designation along with Rare Pediatric Disease Designation from the U.S. Food and Drug Administration (FDA) for the treatment of OTC deficiency. OTC is a key enzyme in the urea cycle which converts toxic ammonia into urea. Elevated ammonia can lead to metabolic crises with progressive and irreversible neurocognitive damage. A safe and effective mRNA therapeutic may restore normal functional OTC enzyme in the liver which could improve urea cycle activity, reduce abnormally elevated glutamine, maintain normal ammonia levels and potentially eliminate the risk of future metabolic crises. ARCT-810 is based on Arcturus' mRNA design construct and proprietary manufacturing process. ARCT-810 also utilizes Arcturus' extensive and propriety lipid library and employs the Company's LUNAR® delivery platform to deliver OTC mRNA to hepatocytes.

About Arcturus

Founded in 2013 and based in San Diego, California, Arcturus Therapeutics Holdings Inc. (Nasdaq: ARCT) is a commercial 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 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 500 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 X (formerly 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), the timing, scope and participants in the KOL event, likelihood of approval of ARCT-810 in the United States or elsewhere, or ARCT-810's potential for therapeutic benefit, 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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