Arcturus Therapeutics to Present Ornithine Transcarbamylase (OTC) Deficiency Phase 2 Clinical Data and Provide Update on mRNA Liver Therapeutics Platform

September 18, 2026

SAN DIEGO--(BUSINESS WIRE)--Sep. 18, 2026-- Arcturus Therapeutics Holdings Inc. (the “Company”, “Arcturus”, Nasdaq: ARCT), a messenger RNA medicines company focused on the development of liver and respiratory rare disease therapeutics, today announced that it will host a virtual presentation on Wednesday, September 23, 2026, at 4:30 p.m. ET.

The presentation will provide an overview of the ARCT-810 Phase 2 clinical program for Ornithine transcarbamylase (OTC) deficiency and Arcturus’ mRNA liver therapeutics platform. Prior to the call, the Company will issue a press release summarizing the presentation.

Marshall Summar, M.D., Former founding member and Executive Committee member of the NIH UCD Consortium, a recognized expert in rare diseases and OTC deficiency, will participate in the presentation.

- 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 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 National Organization for Rare Disorders Lifetime Achievement Award in 2022.

Virtual Presentation: Wednesday, September 23, 2026, at 4:30 p.m. ET

- Domestic: 1-800-347-6865
- International: 1-203-518-9757
- 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 keep 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 proprietary 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 messenger RNA medicines company focused on the development of liver and respiratory rare disease therapeutics 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' strategic collaborations include Thermo Fisher Scientific for ARCT-032 manufacturing and development, BARDA for pandemic influenza initiatives, and ARCALIS, a Japanese joint venture focused on manufacturing mRNA vaccines and therapeutics. Arcturus' pipeline includes RNA therapeutic candidates to potentially treat cystic fibrosis (CF) and ornithine transcarbamylase (OTC) deficiency. Arcturus' versatile RNA therapeutics platforms can be applied toward multiple types of nucleic acid medicines including messenger RNA, small interfering RNA (siRNA), 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. Please connect with us on X and LinkedIn.

View source version on businesswire.com: https://www.businesswire.com/news/home/20260918208396/en/

Arcturus Therapeutics
Public Relations & Investor Relations
Neda Safarzadeh
VP, Head of IR/PR/Marketing
(858) 900-2682
IR@ArcturusRx.com

Source: Arcturus Therapeutics Holdings Inc.