Arcturus Therapeutics Announces Second Quarter 2025 Financial Update and Pipeline Progress

August 11, 2025

Cystic fibrosis (ARCT-032) Phase 2 interim data from first nine participants to be presented in September

Cystic fibrosis Phase 2 trial expected to complete enrollment by year end 2025

OTC deficiency (ARCT-810) Phase 3 trial design alignment with regulatory agencies expected H1 2026

Seasonal flu (ARCT-2138) showed positive Phase 1 results

BARDA pandemic flu (ARCT-2304) Phase 1 results expected 2025

Investor conference call at 4:30 p.m. ET today

SAN DIEGO--(BUSINESS WIRE)--Aug. 11, 2025-- Arcturus Therapeutics Holdings Inc. (the “Company”, “Arcturus”, Nasdaq: ARCT), a commercial messenger RNA medicines company focused on the development of liver and respiratory rare disease therapeutics and infectious disease vaccines, today announced its financial results for the second quarter ended June 30, 2025, and provided corporate updates.

“The Company continues to advance and provide meaningful clinical data across our mRNA therapeutics and vaccines pipeline,” said Joseph Payne, President & CEO of Arcturus Therapeutics. “We are especially pleased with the recent proof-of-concept in our liver platform based on the positive ARCT-810 interim Phase 2 data and look forward to sharing two cohorts of Phase 2 CF data in September.”

Recent Corporate Highlights

- Arcturus is advancing enrollment of adult CF participants in the open label Phase 2 multiple ascending dose CF study (NCT06747858) with daily inhaled treatments of ARCT-032 over a period of 28 days and expects to complete enrollment as planned by year end.
    - All six participants in the second cohort (10 mg) are expected to complete dosing in early September. The Company expects to provide Phase 2 interim data from the first nine enrolled participants (N = 3 @ 5 mg; N = 6 @ 10 mg) in September 2025.
    - The Company anticipates meetings with the FDA and other regulatory agencies in H1 2026 to discuss the Phase 2 data and plans for pivotal trials, including the enrollment of adolescent and pediatric participants, followed by Phase 3 initiation in 2026.
- In June, the company announced positive interim data from two Phase 2 multiple dose studies conducted in the OTC program.
    - In each study and in combined analyses of both Phase 2 studies, decreases in glutamine levels to within normal range were observed following multiple ARCT-810 administrations to participants who remained on their standard of care therapy. Mean ammonia levels were stable within the normal range following at least two doses of ARCT-810 and remained stable for approximately 28 days after completion of dosing.
    - During the treatment phase and follow-up, two out of three participants in the Phase 2 U.S. study (NCT06488313) showed increases in relative ureagenesis function to levels observed in asymptomatic OTC deficient patients (≥ 50% of healthy controls) as measured by a newly developed and optimized 15N-ureagenesis assay. The remaining participant demonstrated increased 15N-citrulline enrichment. The data, taken together, suggest improvement of urea cycle function in all 3 participants.
    - ARCT-810 was generally safe and well tolerated in single dose Phase 1/1b and multi-dose Phase 2 studies, comprising 40 participants to date, including 20 OTC deficient participants.
    - The Company is preparing for meetings with the U.S. FDA and other regulatory agencies to discuss the clinical significance of the observed biomarker changes in relation to the design of the Phase 3 pivotal trial and pediatric studies. Phase 3 biomarker and trial design alignment with regulators is expected in first half of 2026.
- KOSTAIVE® regulatory updates include:
    - A Marketing Authorization Application (MAA) filed by CSL to the UK Medicines and Healthcare Products Regulatory Agency (MHRA) in Q2 2025, approval expected by September 2025.
    - NDA applications filed by Meiji Seika Pharma to Japan’s Pharmaceuticals and Medical Devices Agency (PMDA) for the 2-dose lyophilized vaccine presentation in H1 2025, and the 2025-2026 season's SARS-CoV-2 variant update was completed in Q2 2025, with anticipated approvals in Q3/Q4 2025.

    - U.S. BLA filing to the FDA remains on track for Q3 2025, with an approval decision expected in 2026.
- Under our collaboration with CSL Seqirus, we conducted a Phase 1 study (NCT06125691) of ARCT-2138, an sa-mRNA seasonal influenza vaccine candidate, encoding hemagglutinin (HA) and neuraminidase (NA) of 4 influenza strains recommended by the WHO. The clinical study report was finalized in June 2025. The study objectives were to evaluate the safety and tolerability and to describe the immune response of different dose levels of the vaccine in 100 young adults (18-49 years of age) and 35 older adults (≥ 65 years of age).
    - All tested dose levels of ARCT-2138 were immunogenic against all four influenza strains as measured by hemagglutinin-inhibition assay in both age groups, demonstrating a modest dose-response (≤ 2.1-fold) within the range of the tested doses (2-20 µg).
    - ARCT-2138 also induced NA-specific antibody responses at all tested dose levels of ARCT-2138 against all four influenza strains. The frequencies of unsolicited adverse events and medically attended adverse events were similar to comparator vaccines. No major safety concerns were raised from the study results.
    - Overall, the study showed the potential of a self-amplifying mRNA vaccine, encoding eight antigens, to induce an immune response in both young and older adults with a dose as low as 2 µg, and tolerable up to 20 µg.
- The Company is expecting Phase 1 results in 2025 from ARCT-2304, an sa-mRNA vaccine candidate for Pandemic Influenza A Virus H5N1 which recently received U.S. FDA Fast Track Designation.
    - No safety concerns were raised from available clinical data from the ongoing Phase 1 clinical study (NCT06602531) with 212 participants; all three tested dose levels (1.5, 5, and 12 µg) were well-tolerated, with the majority of the reported solicited AEs being mild-to-moderate severity and short-lived.
    - Immunogenicity results are expected in Q4 2025.
    - This project has been supported in whole with federal funds from the Department of Health and Human Services; Administration for Strategic Preparedness and Response; Biomedical Advanced Research and Development Authority (BARDA), under contract number 75A50122C0007.
- The Company appointed Moncef Slaoui, Ph.D., as Chairman of the Board on July 1, 2025.

Financial Results for the three months ended June 30, 2025

Revenues in conjunction with strategic alliances and collaborations:

Arcturus' primary revenue streams include license fees, consulting and related technology transfer fees, reservation fees and collaborative payments received from research and development arrangements with pharmaceutical and biotechnology partners. Revenue for the three and six months ended June 30, 2025, was $28.3 million and $57.7 million, respectively, representing decreases of $21.6 million and $30.2 million compared to the same periods in 2024. These declines were primarily driven by reduced revenue from the CSL collaboration, reflecting lower supply agreement activity and lower amortization of the upfront payment as KOSTAIVE® progresses toward commercialization.

Operating expenses:

Total operating expenses for the three months ended June 30, 2025, were $39.9 million compared with $71.0 million for the three months ended June 30, 2024. Total operating expenses for the six months ended June 30, 2025, were $86.1 million compared with $139.4 million for the six months ended June 30, 2024.

Research and development expenses:

Research and development expenses consist primarily of external manufacturing costs, in vivo research studies and clinical trials performed by contract research organizations, clinical and regulatory consultants, personnel-related expenses, facility-related expenses and laboratory supplies related to conducting research and development activities. Research and development expenses were $29.6 million for the three months ended June 30, 2025, compared with $58.7 million for the three months ended June 30, 2024. The decrease was primarily driven by lower manufacturing costs for the KOSTAIVE, LUNAR-FLU, and cystic fibrosis programs, and reduced clinical trial expenses for KOSTAIVE and Ornithine Transcarbamylase Deficiency. Lower payroll and employee benefits also contributed to the decrease. These decreases were partially offset by higher clinical costs for cystic fibrosis following the start of Phase 2 trials in fiscal year 2025.

Research and development expenses were $64.5 million for the six months ended June 30, 2025, compared with $112.2 million for the three months ended June 30, 2024. The decrease was primarily driven by lower manufacturing and clinical costs for the KOSTAIVE program, reflecting the program's transition from a development program to the commercial phase. Additional decreases resulted from lower payroll and benefits expenses and reduced facilities and equipment costs following the downsizing of operations. These reductions were partially offset by higher clinical expenses for the cystic fibrosis program.

General and Administrative Expenses:

General and administrative expenses primarily consist of salaries and related benefits for executive, administrative, legal and accounting functions and professional service fees for legal and accounting services as well as other general and administrative expenses. General and administrative expenses were $10.3 million and $21.7 million for the three and six months ended June 30, 2025, respectively, compared with $12.3 million and $27.2 million in the comparable periods last year. The decreases in both periods were primarily due to reduced share-based compensation expense as well as reduced payroll and benefits. We expect general and administrative expenses to continue to decrease slightly during the next twelve months driven by lower share-based compensation costs.

Net Loss:

For the three months ended June 30, 2025, Arcturus reported a net loss of approximately $9.2 million, or ($0.34) per diluted share, compared with a net loss of $17.2 million, or ($0.64) per diluted share in the three months ended June 30, 2024. For the six months ended June 30, 2025, Arcturus

reported a net loss of approximately $23.3 million, or ($0.86) per diluted share, compared with a net loss of $44.0 million, or ($1.64) per diluted share in the six months ended June 30, 2024.

Cash Position and Balance Sheet:

Cash, cash equivalents and restricted cash were $253.4 million as of June 30, 2025, and $293.9 million on December 31, 2024. Based on the current pipeline and programs, the cash runway remains extended into 2028.

Earnings Call: Monday, August 11, 2025 @ 4:30 p.m. ET

- Domestic: 1-800-274-8461
- International: 1-203-518-9814
- Conference ID: ARCTURUS
- Webcast: Link

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-032 and ARCT-810) and partnered programs (including the COVID-19 and flu programs partnered with CSL Seqirus), the likelihood of and timing for providing interim data from the ARCT-032 Phase 2 CF study, the likelihood of and timing for completion of enrollment in the ARCT-032 Phase 2 CF study, the likelihood of and timing for Phase 3 trial design alignment with regulatory agencies for ARCT-810, the timing for Phase 1 results from the BARDA pandemic flu Phase 1 study, the likelihood of and timing for meetings with the FDA and other regulatory agencies relating to the CF and OTC programs, the likelihood of and timing for initiation of Phase 3 studies for the CF and OTC programs, the timing for completion of enrollment in the ARCT-032 (CF) Phase 2 study, the likelihood of and timing for approval of the MAA on KOSTAIVE filed by CSL to the UK MHRA, the likelihood and timing for approvals of NDA applications for KOSTAIVE filed by Meiji Seika Pharma with Japan's PMDA, the planned U.S. BLA filing and expected approval decision for KOSTAIVE, efforts for optimization and testing for seasonal influenza program, the timing for Phase 1 results for the pandemic influenza vaccine candidate, the likelihood that general and administrative expenses will decrease, the likelihood that preclinical or clinical data will be predictive of future clinical results, its current cash position and expected cash burn and runway, 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.

ARCTURUS THERAPEUTICS HOLDINGS INC. AND ITS SUBSIDIARIES
CONDENSED CONSOLIDATED BALANCE SHEETS

(in thousands, except par value information)	June 30, 2025 (unaudited)	December 31, 2024
Assets		
Current assets:		
Cash and cash equivalents	$ 196,467	$ 237,028
Restricted cash	55,000	55,000
Accounts receivable	17,204	3,974
Prepaid expenses and other current assets	5,832	9,977
Total current assets	274,503	305,979
Property and equipment, net	8,088	9,531
Operating lease right-of-use assets, net	24,794	26,674
Non-current restricted cash	1,885	1,885
Total assets	$ 309,270	$ 344,069

Liabilities and stockholders' equity		
Current liabilities:		
Accounts payable	$ 10,623	$ 7,194
Accrued liabilities	24,629	38,781
Deferred revenue	11,263	19,514
Total current liabilities	46,515	65,489
Deferred revenue, net of current portion	8,769	12,604
Operating lease liability, net of current portion	22,933	24,998
Total liabilities	78,217	103,091
Stockholders' equity		
Common stock, $0.001 par value; 60,000 shares authorized; issued and outstanding shares were 27,148 at June 30, 2025 and 27,096 at December 31, 2024	27	27
Additional paid-in capital	703,089	689,758
Accumulated deficit	(472,063)	(448,807)
Total stockholders' equity	231,053	240,978
Total liabilities and stockholders' equity	$ 309,270	$ 344,069

ARCTURUS THERAPEUTICS HOLDINGS INC. AND ITS SUBSIDIARIES
CONDENSED CONSOLIDATED STATEMENTS OF OPERATIONS AND COMPREHENSIVE LOSS
(unaudited)

	Three Months Ended June 30,		Six Months Ended June 30,	
(in thousands, except per share data)	**2025**	**2024**	**2025**	**2024**
Revenue:				
Collaboration revenue	$ 24,510	$ 45,976	$ 49,987	$ 78,574
Grant revenue	3,791	3,883	7,696	9,297
Total revenue	28,301	49,859	57,683	87,871
Operating expenses:				
Research and development, net	29,579	58,669	64,471	112,242
General and administrative	10,338	12,316	21,654	27,167
Total operating expenses	39,917	70,985	86,125	139,409
Loss from operations	(11,616)	(21,126)	(28,442)	(51,538)
Loss from foreign currency	(127)	(388)	(149)	(441)
Finance income, net	2,567	4,148	5,339	8,164
Net loss before income taxes	(9,176)	(17,366)	(23,252)	(43,815)
Provision (benefit) for income taxes	4	(150)	4	218
Net loss	$ (9,180)	$ (17,216)	$ (23,256)	$ (44,033)
Net loss per share, basic and diluted	$ (0.34)	$ (0.64)	$ (0.86)	$ (1.64)
Weighted-average shares outstanding, basic and diluted	27,129	26,967	27,118	26,923
Comprehensive loss:				
Net loss	$ (9,180)	$ (17,216)	$ (23,256)	$ (44,033)
Comprehensive loss	$ (9,180)	$ (17,216)	$ (23,256)	$ (44,033)

View source version on businesswire.com: https://www.businesswire.com/news/home/20250811954049/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.