Arcturus Therapeutics Announces Third Quarter 2024 Financial Update and Pipeline Progress

November 7, 2024

Cystic Fibrosis and OTC Deficiency Phase 2 studies on track for POC data in first half of 2025

$25 Million commercial milestone achieved with first sale of KOSTAIVE® in Japan

KOSTAIVE® European CHMP opinion expected December

Positive results from multiple Phase 3 studies support KOSTAIVE® U.S. BLA filing in H1 2025

Superior 12-month durability results from Phase 3 study of KOSTAIVE® published in The Lancet Infectious Diseases

Positive Phase 3 results showed KOSTAIVE® XBB.1.5. met all four primary study objectives and key secondary objectives

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

SAN DIEGO--(BUSINESS WIRE)--Nov. 7, 2024-- 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 its financial results for the third quarter ended September 30, 2024, and provided corporate updates.

“I am thrilled about the approval of KOSTAIVE® for the COVID-19 JN.1 strain in Japan and for the continued success of the STARR® platform in multiple Phase 3 trials, underscoring CSL and Arcturus’ commitment to deliver disruptive technologies for protection against respiratory viral diseases,” said Joseph Payne, President & CEO of Arcturus Therapeutics. “I am pleased that both of our flagship mRNA therapeutic programs, ARCT-032 and ARCT-810, are on track for interim Phase 2 proof-of-concept clinical data in the first half of 2025. These studies allow us to evaluate lung function improvement in individuals with cystic fibrosis, and meaningful biomarker changes in individuals with OTC deficiency.”

“I am happy to report our first commercial milestone achieved from our CSL partnership for the first commercial sale of KOSTAIVE in Japan,” said Andy Sassine, Chief Financial Officer of Arcturus. “We anticipate another milestone related to potential European approval in the first quarter of 2025. I am also happy to announce that Arcturus is planning to transfer our cystic fibrosis manufacturing process technology to ARCALIS.”

Recent Corporate Highlights

- In September, Arcturus received clearance of an Investigational New Drug application from the U.S. Food and Drug Administration (FDA), enabling the Company to initiate a Phase 2 multiple ascending dose study to evaluate the safety, tolerability and efficacy of ARCT-032 in people with cystic fibrosis (CF).
    - The Phase 2 study is screening individuals with CF who do not qualify for, or benefit from, CFTR modulator medicines due to dysfunctional or absent CFTR protein and/or drug intolerance.
    - The Company remains on track to share ARCT-032 Phase 2 proof-of-concept (POC) interim data in 1H25.
- In August, the Company announced the expansion of the Phase 2 clinical program of ARCT-810, an mRNA therapeutic to potentially treat ornithine transcarbamylase (OTC) deficiency, into the United States.
    - This open-label multiple-dose study (NCT06488313) evaluating pharmacodynamics and safety is currently enrolling adults and adolescents requiring clinical management for OTC deficiency.
    - The placebo-controlled Phase 2 European study has completed the dosing phase (N = 8; 0.3 mg/kg) in OTC deficient individuals.
    - The Company remains on track to share ARCT-810 Phase 2 POC interim data from both U.S. and European studies in 1H25.
- Meiji Seika Pharma, CSL’s exclusive partner in Japan, began KOSTAIVE commercial sales in September 2024. This event triggered a $25 million commercial milestone associated with the first sale of KOSTAIVE® in Japan.
- In September, the Company, along with partners CSL and Meiji, announced new 12-month post vaccination data for KOSTAIVE at OPTIONS XII for the Control of Influenza conference.
    - The results of a head-to-head study demonstrated that KOSTAIVE maintained superior immunogenicity compared to the conventional mRNA vaccine COMIRNATY® for up to one year against Wuhan-Hu-1, Omicron BA.4/5 and certain other variants, and at one-sixth the dose of the comparator (5 µg vs 30 µg, respectively). The results were published in The Lancet Infectious Disease.
    - Additional Phase 3 data presented by CSL, Meiji and Arcturus show that bivalent KOSTAIVE, ARCT-2301, induced superior immunogenicity over conventional bivalent mRNA vaccine COMIRNATY® that persists against key variants up to six months post vaccination.

- Earlier this year, CSL Seqirus's partner Meiji Seika Pharma announced that it submitted a partial change application for an amendment to the manufacturing and marketing approval of KOSTAIVE® to include manufacturing sites in Japan, including ARCALIS, Inc., Arcturus' manufacturing joint venture in Japan. When approved, Meiji Seika Pharma will begin selling domestically produced KOSTAIVE® this season.
- The Company announced the results of a Phase 3 study which demonstrated the added value of an updated COVID-19 vaccine (ARCT-2303) containing the Omicron XBB.1.5 variant. The study supports co-administration of KOSTAIVE with licensed influenza vaccines.
    - ARCT-2303 demonstrated superior immune response versus ARCT-154 as measured by neutralizing antibodies against Omicron XBB.1.5.6 in terms of GMT ratio and SCR difference.
    - Co-administration of ARCT-2303 and cell-based quadrivalent influenza vaccine (QIV; FLUCELVAX®, CSL) showed noninferior immune response vs standalone QIV administration.
    - Co-administration of ARCT-2303 and QIV showed noninferior immune response vs standalone ARCT-2303 administration.
    - Co-administration of ARCT-2303 and adjuvanted QIV (FLUAD®, CSL) in older adults showed similar responses vs standalone administration of ARCT-2303 and adjuvanted QIV.

Financial Results for the three months ended September 30, 2024

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. For the three months ended September 30, 2024, we reported revenue of $41.7 million, a slight decrease of $3.5 million from the $45.2 million reported in the same period in 2023. The decline was mostly attributable to a lower milestone achievement from the CSL agreement during the third quarter of 2024. This decrease was offset by revenue recognized from a supply agreement related to the commercial production of KOSTAIVE® and an increase in revenue from the BARDA agreement during the three months ended September 30, 2024.

Revenue decreased by $6.4 million during the nine months ended September 30, 2024, as compared to the same period in 2023. The decrease was due to lower CSL revenue resulting from the timing and value of milestone achievements. This was offset by increased BARDA revenue due to progress of the pandemic flu program.

Operating expenses:

Total operating expenses for the three months ended September 30, 2024, were $52.4 million compared with $64.5 million for the three months ended September 30, 2023. Total operating expenses for the nine months ended September 30, 2024, were $191.8 million compared with $195.9 million for the nine months ended September 30, 2023.

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 $39.1 million for the three months ended September 30, 2024, compared with $51.1 million for the three months ended September 30, 2023. Research and development expenses were $151.4 million for the nine months ended September 30, 2024, compared with $155.5 million for the nine months ended September 30, 2023. The decreases in research and development expenses were primarily driven by a decrease in manufacturing costs for the COVID program. The decrease was partially offset by an increase in clinical trial costs for the COVID and flu programs.

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 $13.3 million and $40.4 million for the three and nine months ended September 30, 2024, respectively, compared with $13.4 million and $40.4 million for the comparable periods in the prior year. These expenses remained relatively consistent between the two periods. The Company expects that general and administrative expenses will remain relatively consistent over the next fiscal year with the current pipeline.

Net Loss:

For the three months ended September 30, 2024, Arcturus reported a net loss of approximately $6.9 million, or ($0.26) per diluted share, compared with a net loss of $16.2 million, or ($0.61) per diluted share in the three months ended September 30, 2023. For the nine months ended September 30, 2024, Arcturus reported a net loss of approximately $50.9 million, or ($1.89) per diluted share, compared with a net loss of $18.0 million, or ($0.68) per diluted share in the nine months ended September 30, 2023.

Cash Position and Balance Sheet:

Cash, cash equivalents and restricted cash were $294.1 million as of September 30, 2024, and $348.9 million on December 31, 2023. Arcturus achieved a total of approximately $462.1 million in upfront payments and milestones from CSL as of September 30, 2024, and expects to continue to receive future milestone payments from CSL supporting the ongoing development of the COVID and flu programs and three additional vaccine programs by CSL. Based on the current pipeline and programs, the cash runway is expected to extend through the first quarter of fiscal year 2027.

Earnings Call: Thursday, November 7, 2024 @ 4:30 pm 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 ornithine transcarbamylase (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 400 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 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 and continued advancement 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 and extent of commercialization of KOSTAIVE and the timing thereof, the continued clinical development of the rare disease programs, the interim Phase 2 proof-of-concept clinical data and the timing therefor, the likelihood and timing of European Marketing Authorization application approval for KOSTAIVE and of a milestone payment from CSL related thereto, the planned transfer of the CF manufacturing process to ARCALIS and timing thereof, the anticipated enrollment in the Phase 2 clinical program for ARCT-810, the anticipated enrollment in the Phase 2 clinical program for ARCT-032, that preclinical or clinical data will be predictive of future clinical results, the likelihood and timing of clinical study updates, the likelihood of and timing for approval of Meiji Seika Pharma's application to amend approval for KOSTAIVE to include domestic manufacturing sites in Japan, Meiji Seika Pharma's plans to begin selling Japan-produced KOSTAIVE and the timing thereof, the likelihood or timing of collection of accounts receivables including expected future milestone and other payments from CSL, 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.

Trademark Acknowledgements

The Arcturus logo and other trademarks of Arcturus appearing in this announcement, including LUNAR® and STARR®, are the property of Arcturus. All other trademarks, services marks, and trade names in this announcement are the property of their respective owners.

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

(in thousands, except par value information)	September 30, 2024 (unaudited)	December 31, 2023
Assets		
Current assets:		
Cash and cash equivalents	$ 237,178	$ 292,005
Restricted cash	55,000	55,000
Accounts receivable	30,199	32,064
Prepaid expenses and other current assets	8,444	7,521
Total current assets	330,821	386,590
Property and equipment, net	10,350	12,427
Operating lease right-of-use assets, net	27,598	28,500
Non-current restricted cash	1,885	1,885
Total assets	$ 370,654	$ 429,402
Liabilities and stockholders' equity		
Current liabilities:		
Accounts payable	$ 10,131	$ 5,279
Accrued liabilities	32,396	31,881
Deferred revenue	26,936	44,829
Total current liabilities	69,463	81,989

Deferred revenue, net of current portion	13,338	42,496
Operating lease liability, net of current portion	25,987	25,907
Other non-current liabilities	—	497
Total liabilities	108,788	150,889
Stockholders' equity		
Common stock, $0.001 par value; 60,000 shares authorized; issued and outstanding shares were 27,084 at September 30, 2024 and 26,828 at December 31, 2023	27	27
Additional paid-in capital	680,641	646,352
Accumulated deficit	(418,802)	(367,866)
Total stockholders' equity	261,866	278,513
Total liabilities and stockholders' equity	$ 370,654	$ 429,402

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

	Three Months Ended September 30,		Nine Months Ended September 30,	
(in thousands, except per share data)	2024	2023	2024	2023
Revenue:				
Collaboration revenue	$ 38,815	$ 43,376	$ 117,389	$ 132,670
Grant revenue	2,858	1,764	12,155	3,274
Total revenue	41,673	45,140	129,544	135,944
Operating expenses:				
Research and development, net	39,134	51,077	151,376	155,513
General and administrative	13,276	13,377	40,443	40,364
Total operating expenses	52,410	64,454	191,819	195,877
Loss from operations	(10,737)	(19,314)	(62,275)	(59,933)
(Loss) gain from foreign currency	(201)	4	(642)	(175)
Gain on debt extinguishment	—	—	—	33,953
Finance income, net	3,818	3,981	11,981	9,710
Net loss before income taxes	(7,120)	(15,329)	(50,936)	(16,445)
Provision for income taxes	(217)	893	—	1,573
Net loss	$ (6,903)	$ (16,222)	$ (50,936)	$ (18,018)
Net loss per share, basic and diluted	$ (0.26)	$ (0.61)	$ (1.89)	$ (0.68)
Weighted-average shares outstanding, basic and diluted	27,062	26,574	26,970	26,559
Comprehensive loss:				
Net loss	$ (6,903)	$ (16,222)	$ (50,936)	$ (18,018)
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