Arcturus Therapeutics Announces First Quarter 2021 Company Overview and Financial Results and Provides New Clinical Data

May 10, 2021

ARCT-021 Phase 2 interim data showed favorable safety profile and greater than 90% seroconversion after a single dose; data supports advancement into Phase 3

In negotiations with multiple regulatory authorities regarding ARCT-021 Phase 3 study program

Continued progress advancing mRNA therapeutic platform, including liver (ARCT-810) and lung (ARCT-032) targeted programs

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

SAN DIEGO,--(BUSINESS WIRE)--May 10, 2021-- Arcturus Therapeutics Holdings Inc. (the “Company”, “Arcturus”, Nasdaq: ARCT), a leading clinical-stage messenger RNA medicines company focused on the development of infectious disease vaccines and significant opportunities within liver and respiratory rare diseases, today announced its financial results for the first quarter ended March 31, 2021 and provided corporate updates.

“Arcturus has made continued progress advancing our clinical pipeline, highlighted by our development efforts for ARCT-021, a differentiated mRNA vaccine candidate targeting COVID-19. Based on promising data from our Phase 1/2 study, and the new interim safety and immunogenicity data from our Phase 2 study, we are looking forward to initiating ARCT-021 Phase 3 study as well as the continued advancement of our other pipeline programs,” said Steve Hughes, M.D., Chief Medical Officer of Arcturus.

“Our Self-Transcribing and Replicating mRNA (STARR™) delivered with our LUNAR® delivery system potentially has important differences from currently authorized mRNA vaccines for emergency use. We believe that ARCT-021, as a single shot lyophilized self-amplifying mRNA vaccine, if approved, may represent a preferred vaccine option. We are very pleased with the recent successes in our mRNA drug product manufacturing efforts, and in the expansion of our global safety database – and the positive readthrough this provides to the Company’s pipeline of mRNA therapeutics. We eagerly look forward to the next major development milestones for our vaccine, liver, and lung mRNA pipeline programs,” said Joseph Payne, President & CEO of Arcturus.

Recent Corporate Highlights

ARCT-021, STARR™ mRNA Vaccine Candidate for SARS-CoV-2

Phase 2 study:

- Completed enrollment with 580 participants randomized and dosed
- Completed two interim analyses of the Phase 2 data which have been reviewed by the Data and Safety Monitoring Board (DSMB) with recommendation to proceed with no changes to protocol
- Data support initiation of Phase 3 study evaluating a single shot regimen of 5 microgram (µg)
- Interim immunogenicity data confirm high seroconversion rate (> 90%) at day 28 for IgG antibodies binding the full-length spike protein following a single 5 µg dose
- Phase 2 study is ongoing and the Company remains blinded to full trial data; data from additional endpoints are expected, including neutralizing antibody and T-cell data, during H2 2021

Phase 3 study:

- Single dose of 5 µg selected as the regimen for evaluation in Phase 3
- In negotiations with multiple regulatory authorities regarding Phase 3 study

Manufacturing:

- Completed GMP manufacturing and release of lyophilized ARCT-021 to support Phase 3
- Completed stockpiling of greater than 10 million doses of lyophilized ARCT-021
- Manufacturing capacity in place to support vaccine supply requirements under potential Emergency Use Authority (EUA)
- Lyophilized ARCT-021 stability predicted to be longer than 1 year at -20°C; stability studies at -20°C, 2-8°C and room temperature are ongoing

ARCT-810, mRNA Therapeutic Candidate for Ornithine Transcarbamylase (OTC) Deficiency

- Two additional sites in U.S. activated to support ongoing Phase 1b study

- 9-month (20 doses) chronic toxicology study completed in non-human primates with no adverse histological findings; doses exceeded maximum targeted clinical dose
- On track to file CTA this quarter for Phase 2 multiple dose study; ethics committee submission completed

ARCT-032, mRNA Therapeutic Candidate for Cystic Fibrosis

- CTA filing on track for Q4

Financial results for the quarter ended March 31, 2021

Revenues in conjunction with strategic alliances and collaborations: Arcturus' primary sources of revenues were from license fees and collaborative payments received from research and development arrangements with pharmaceutical and biotechnology partners. For the three months ended March 31, 2021, the Company reported revenue of $2.1 million, compared with $2.6 million in the three months ended March 31, 2020.

Operating expenses: Total operating expenses for the three months ended March 31, 2021 were $59.8 million compared with $12.1 million for the three months ended March 31, 2020 and $33.3 million for the three months ended December 31, 2020.

Research and development expenses increased by approximately $42.1 year over year and $25.8 million sequentially from the fourth quarter of 2020. The primary driver of the sequential increase was driven by higher clinical and manufacturing cost of $17.2 million for our ARCT-021 program. The remaining increase was primarily driven by higher personnel costs and related to the acquisition of an exclusive license from Alexion Pharmaceuticals for certain patent-pending inventions relating to nucleic acid purification technologies for approximately $5.0 million of Arcturus stock.

Net loss: For the three months ended March 31, 2021 Arcturus reported a net loss of approximately $56.3 million, or ($2.15) per basic and diluted share, compared with a net loss of $9.8 million, or ($0.67) per basic and diluted share in the three months ended March 31, 2020 and a net loss of $31.1 million, or ($1.25) per basic and diluted share in the three months ended December 31, 2020.

The Company's cash balance totaled $466.9 million as of March 31, 2021, compared to cash and cash equivalents of $463.0 million at December 31, 2020. Based on our current plans, the Company's cash position is expected to be sufficient to support operations for more than two years.

Monday, May 10th @ 4:30 p.m. ET

Domestic:	877-256-4295
International:	212-231-2927
Conference ID:	21993793
Webcast:	http://public.viavid.com/index.php?id=144682

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 and vaccine candidates includes mRNA vaccine programs for SARS-CoV-2 (COVID-19) and Influenza, and other programs to potentially treat Ornithine Transcarbamylase (OTC) Deficiency, and Cystic Fibrosis along with partnered programs including Glycogen Storage Disease Type 3, Hepatitis B Virus, and non-alcoholic steatohepatitis (NASH). 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 (209 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 Medical School, and the Cystic Fibrosis Foundation. 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, collaborations, the planned initiation, design or completion of clinical trials, the likelihood that the Company will obtain clearance from regulatory authorities to proceed with planned clinical trials, the likelihood that preclinical or clinical data will be predictive of future clinical results, the likelihood that clinical data will be sufficient for regulatory approval or completed in time to submit an application for regulatory approval within a particular timeframe, the likelihood of success (including safety and efficacy) of the Company's pipeline, including ARCT-021, ARCT-810 or ARCT-032, the potential administration regimen or dosage, or ability to administer multiple doses of, any of Company's drug candidates, the ability to enroll subjects in clinical trials, the Company's efforts to develop a vaccine against COVID-19 and therapeutic potential thereof based on the Company's mRNA therapeutics, the Company's manufacturing methods and technologies (including purification and lyophilization, and stability of manufactured product), the ability of the Company to scale up manufacturing of products or substances, the likelihood that a patent will issue from any patent application, its current cash position and expected cash burn and the impact of general business and economic conditions. Actual results and performance could differ materially from those projected in any forward-looking statements as a result of many factors including, without limitation, the ability to enroll subjects in clinical trials as a result of the COVID-19 pandemic, the impact of commercialization of third-party COVID-19 vaccines on the design, and ability to conduct, clinical trials, the availability of manufacturing capacity and raw materials, unexpected clinical results, government regulations impacting the regulatory environment or intellectual property landscape, and general market conditions that may prevent such achievements or performance. 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, 2020, and in subsequent filings with, or submissions to, the SEC. 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)

	March 31, 2021	December 31, 2020
	(unaudited)	
Assets		
Current assets:		
Cash and cash equivalents	$ 466,839	$ 462,895
Accounts receivable	2,007	2,125
Prepaid expenses and other current assets	1,150	2,769
Total current assets	469,996	467,789
Property and equipment, net	3,427	3,378
Operating lease right-of-use asset, net	6,690	5,182
Equity-method investment	1,248	—
Non-current restricted cash	107	107
Total assets	$ 481,468	$ 476,456
Liabilities and stockholders' equity		
Current liabilities:		
Accounts payable	$ 5,597	$ 10,774
Accrued liabilities	29,800	20,639
Deferred revenue	17,936	18,108
Total current liabilities	53,333	49,521
Deferred revenue, net of current portion	11,313	12,512
Long-term debt, net of current portion	58,147	13,845
Operating lease liability, net of current portion	5,710	4,025
Other long-term liabilities	358	—
Total liabilities	$ 128,861	$ 79,903
Stockholders' equity		
Common stock: $0.001 par value; 60,000 shares authorized; 26,319 issued and outstanding at March 31, 2021 and 26,192 issued and outstanding at December 31, 2020	26	26
Additional paid-in capital	552,743	540,343
Accumulated deficit	(200,162)	(143,816)
Total stockholders' equity	352,607	396,553
Total liabilities and stockholders' equity	$ 481,468	$ 476,456

ARCTURUS THERAPEUTICS HOLDINGS INC. AND ITS SUBSIDIARIES
CONDENSED CONSOLIDATED STATEMENTS OF OPERATIONS AND COMPREHENSIVE LOSS
(unaudited)
(in thousands except per share data)

	Three Months Ended March 31,		December 31,
	2021	2020	2020
Collaboration revenue	$ 2,127	$ 2,646	$ 2,238
Operating expenses:			
Research and development, net	50,050	7,917	24,286
General and administrative	9,743	4,191	9,034
Total operating expenses	59,793	12,108	33,320
Loss from operations	(57,666)	(9,462)	(31,082)
Gain (loss) from equity-method investment	1,248	(163)	—
Gain from foreign currency	430	—	16
Finance expense, net	(358)	(152)	(38)
Net loss	$ (56,346)	$ (9,777)	$ (31,104)
Net loss per share, basic and diluted	$ (2.15)	$ (0.67)	$ (1.25)
Weighted-average shares outstanding, basic and diluted	26,243	14,521	24,886
Comprehensive loss:			
Net loss	$ (56,346)	$ (9,777)	$ (31,104)
Comprehensive loss	$ (56,346)	$ (9,777)	$ (31,104)

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