



Arcturus Therapeutics Announces Third Quarter 2020 Financial Results and Positive Clinical Updates for ARCT-810 Phase I Study and Additional ARCT-021 Interim Data

November 9, 2020

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

SAN DIEGO--(BUSINESS WIRE)--Nov. 9, 2020-- 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 quarter and nine months ended September 30, 2020, and provided a corporate update.

"This has been an exciting period for Arcturus highlighted by our preliminary ARCT-021 Phase 1/2 COVID-19 vaccine study results. These data, as well as those from our ARCT-810 Phase 1 study, illustrate a highly productive period of clinical advancement of our novel mRNA based therapeutic candidates. The ARCT-021 Phase 1/2 data provide clinical validation for our unique vaccine candidate and suggest that our self-replicating mRNA-based investigational vaccine could have a differentiated clinical profile and may allow vaccination at low doses. We believe that ARCT-021 could play an important role in the massive global vaccination campaigns that will be necessary to control the COVID-19 pandemic. We look forward to rapidly moving ARCT-021 forward in later stage clinical studies," said Joseph Payne, President & CEO of Arcturus.

"The initial ARCT-810 Phase 1/2 study results, showing good tolerability and favorable pharmacokinetics, provide support for the further development of this drug candidate in Ornithine Transcarbamylase (OTC) deficiency, a severe monogenic rare disease where new medicines are urgently needed. These data also provide additional validation for the systemic administration of Arcturus' novel LUNAR technology. We believe that we have developed a powerful and broadly applicable mRNA delivery approach that may be useful for the treatment of numerous serious diseases, and we look forward to continuing to advancing the development of our investigational therapeutics," said Pad Chivukula, Ph.D., Chief Scientific Officer and Chief Operating Officer of Arcturus.

Highlights

ARCT-021, Vaccine Candidate for SARS-CoV-2

- Announced interim Phase 1/2 data; favorable immunogenicity and safety results for both single-dose and prime-boost regimens
- Observed 100% seroconversion for IgG binding antibodies in younger adults; 1 out of 5 older adult participants has not yet seroconverted; observed GMT > 2300 in all cohorts
- Doses to advance to later stage clinical trials include single dose 7.5 µg (GMT > 15,000 for younger adults and > 2300 in older adults at this dose)
- Published preclinical data, including successful challenge model data
- Secured manufacturing slots with Recipharm to support cGMP manufacture; lyophilization technology transfer completed

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

- Completed dose escalation of all cohorts (0.1, 0.2, 0.3, 0.4 mg/kg) in Phase 1 Study
- Commenced enrollment in Phase 1b study in OTC-deficient patients in U.S.; first subject in screening

Agreements

- Executed Definitive Supply Agreement with the Israeli Ministry of Health; up to \$275 million
- Executed Agreement with Singapore; \$45 million limited recourse loan facility and up to \$175 million in vaccine purchases

Financial results for the quarter ended September 30, 2020

Revenues in conjunction with strategic alliances and collaborations: Arcturus' primary source of revenues is from license fees and collaborative payments received from research and development arrangements with pharmaceutical and biotechnology partners. For the three months ended September 30, 2020, the Company reported revenue of \$2.3 million, compared with \$3.3 million in the three months ended September 30, 2019. The decline in collaboration revenues primarily relates to a \$0.8 million decrease in reimbursements from CureVac associated with the OTC collaboration that ended in the third quarter of 2019.

Operating expenses: Total operating expenses for the three months ended September 30, 2020 were \$23.3 million compared with \$10.9 million for the same period of 2019. The current quarter operating expenses were partially offset with \$3.7 million of funds earned under the Singapore vaccine grant and \$0.7 million in funds awarded by the CF Foundation. Research and Development expenses increased approximately \$10 million sequentially from the June 30, 2020 quarter driven primarily by an approximate increase of \$4 million in each of our Lunar-OTC (ARCT-810) and Lunar-Covid19

(ARCT-021) programs mostly due to clinical and manufacturing expenses. Additionally, approximately \$2 million was driven by increased personnel expenses and costs of our two new pipeline programs Lunar-Lung and Lunar CV.

Net loss: For the three months ended September 30, 2020, Arcturus reported a net loss of approximately \$21.0 million, or (\$0.92) per basic and diluted share, compared with a net loss in the three months ended September 30, 2019 of \$7.4 million, or (\$0.56) per basic and diluted share.

Our cash balance totaled \$307.1 million as of the end of Q3, compared to cash and cash equivalents of \$71.5 million at December 31, 2019. The increase in cash, cash equivalents, and investments is primarily due to successfully raising approximately \$262.1 million in net proceeds through two public equity offerings in 2020. Based on our current pipeline, the Company's cash position is expected to be sufficient to support operations for more than two years.

Monday, Nov 9th @ 4:30 p.m. ET

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 International: 201-689-8560
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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 self-replicating mRNA vaccine programs for SARS-CoV-2 (COVID-19) and Influenza, and other programs to potentially treat Ornithine Transcarbamylase (OTC) Deficiency, Cystic Fibrosis, Cardiovascular Disease 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 (200 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, 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, including those regarding strategy, future operations, collaborations, the likelihood of success, and the efficacy or safety, of our pipeline, including ARCT-021 or ARCT-810, the ability to initiate or complete preclinical and clinical development programs, including as a result of the COVID-19 pandemic, the supply and delivery of any product or substance, the likelihood that clinical data will be predictive of future clinical results or sufficient for regulatory approval, 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 ability of the Company to scale up manufacturing of vaccine doses, the amount and timing of any drawn down loan amounts with the EDB, our current cash position and expected cash burn and the impact of general business and economic conditions are forward-looking statements. 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, 2019, filed with the SEC on March 16, 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)

	September 30, 2020	December 31, 2019
	(unaudited)	
Assets		
Current assets:		
Cash and cash equivalents	\$ 307,023	\$ 71,353
Accounts receivable	2,447	2,179
Prepaid expenses and other current assets	4,630	758
Total current assets	314,100	74,290
Property and equipment, net	3,451	2,349
Operating lease right-of-use asset, net	4,862	5,134
Equity-method investment	—	263
Non-current restricted cash	107	107
Total assets	\$ 322,520	\$ 82,143
Liabilities and stockholders' equity		
Current liabilities:		

Accounts payable	\$	6,478	\$	5,793
Accrued liabilities		15,838		7,134
Deferred revenue		5,698		8,397
Total current liabilities		28,014		21,324
Deferred revenue, net of current portion		13,645		15,182
Long-term debt		15,076		14,995
Operating lease liability, net of current portion		4,155		4,850
Total liabilities	\$	60,890	\$	56,351
Stockholders' equity				
Common stock: \$0.001 par value; 30,000 shares authorized; 24,473 and 15,138 issued and outstanding at September 30, 2020 and December 31, 2019, respectively.		25		15
Additional paid-in capital		374,317		97,445
Accumulated deficit		(112,712)		(71,668)
Total stockholders' equity		261,630		25,792
Total liabilities and stockholders' equity	\$	322,520	\$	82,143

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		Nine Months Ended	
	September 30,		September 30,	
	2020	2019	2020	2019
Collaboration revenue	\$ 2,333	\$ 3,318	\$ 7,301	\$ 17,821
Operating expenses:				
Research and development, net	17,699	7,053	33,560	21,646
General and administrative	5,572	3,881	14,183	10,871
Total operating expenses	23,271	10,934	47,743	32,517
Loss from operations	(20,938)	(7,616)	(40,442)	(14,696)
Loss from equity-method investment	—	303	(263)	15
Finance expense, net	(66)	(120)	(339)	(321)
Net loss	\$ (21,004)	\$ (7,433)	\$ (41,044)	\$ (15,002)
Net loss per share, basic and diluted	\$ (0.92)	\$ (0.56)	\$ (2.19)	\$ (1.33)
Weighted-average shares outstanding, basic and diluted	22,938	13,201	18,766	11,248
Comprehensive loss:				
Net loss	\$ (21,004)	\$ (7,433)	\$ (41,044)	\$ (15,002)
Comprehensive loss	\$ (21,004)	\$ (7,433)	\$ (41,044)	\$ (15,002)

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