Arcturus Therapeutics Announces Fourth Quarter and Full Year 2020 Financial Results and Positive Clinical Updates

ARCT-021 single-shot COVID-19 STARR™ mRNA vaccine to be advanced to Phase 3 clinical development – on track to initiate Phase 3 study in Q2

ARCT-021 single shot immunogenicity profile compares favorably with new data generated from recipients of a single dose of an approved conventional mRNA vaccine

ARCT-810 mRNA therapeutic for ornithine transcarbamylase deficiency to be advanced to Phase 2 clinical development – on track to file CTA for Phase 2 multiple dose study in Q2

ARCT-032 mRNA therapeutic for cystic fibrosis: completed successful pre-IND interaction with FDA – on track to file CTA in Q4

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

San Diego, Calif, March 1, 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 quarter and full year ended December 31, 2020 and provided a corporate update.

"Based on highly promising clinical data from our Phase 1/2 study and emerging mRNA vaccine immunological data, we are advancing ARCT-021 for further development in Phase 3. We are presently preparing to move forward a 5 µg single dose regimen, to be confirmed based on pending Phase 2 data. Our self-amplifying mRNA-based investigational vaccine may provide a differentiated clinical profile and characteristics that support widespread distribution across the globe. Our expectation is that successful protection from COVID-19 will require repeated vaccination of billions of individuals for years to come and that ARCT-021 will be re-dosable. We believe that a re-dosable, more easily distributable single shot mRNA vaccine would be a valuable option for many countries," said Joseph Payne, President and CEO of Arcturus.

"In addition to advancing our vaccine franchise, we have made continued progress advancing our pipeline of promising liver and lung mRNA therapeutic candidates. After successfully beginning enrollment in the U.S. for our Phase 1b study for ARCT-810, a therapeutic candidate for Ornithine Transcarbamylase (OTC) Deficiency, we have now also received approval from Health Canada to enroll subjects. We look forward to obtaining clinical data this year," concluded Mr. Payne.

Recent Corporate Highlights

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

1) **Ongoing Phase 2 clinical study and planning for Phase 3 development:**
    - More than 500 participants dosed across USA and Singapore

- Phase 3 study initiation remains on track for Q2
- Targeting application for Emergency Use Authorization in one or more jurisdictions in H2 2021

2) **Encouraging new supportive data from Duke-NUS Medical School submitted for publication:**
   - Dr. Eng Eong Ooi, Professor of Emerging Infectious Diseases at Duke-NUS Medical School, and colleagues, examined the adaptive immune responses of an approved conventional mRNA vaccine after a single administration
   - Data suggest that binding antibodies and cellular immunity are associated with COVID-19 protection at early timepoints after the first injection
   - ARCT-021 single shot immunogenicity profile in Phase 1/2 study compares favorably, providing additional support for the potential efficacy of the ARCT-021 vaccine

	Day	**BNT162b2** (30 µg); N = 20	**ARCT-021** (5 µg); N = 22
IgG (≥ 4-fold rises)	Day 10	**80%**	--
	Day 14	--	**81%***
PRNT (% Detectable)	Day 10	**10%**	--
	Day 28	--	**59%**
ELISpot (SFUs) *Median change from baseline*	Day 10	**28**	--
	Day 15	--	**211**
	Day 21	**13**	--

*Increases to 100% by Day 36
IgG = Immunoglobulin G binding antibodies to full length spike protein
PRNT = plaque reduction neutralization test
SFUs = Spot Forming Units per million peripheral blood mononuclear cells

3) **Completed Phase 1/2 clinical study and supporting preclinical studies:**
   - Phase 1/2 study is complete and final data are under analysis; plan to submit results for publication in Q2
   - ARCT-021 demonstrated robust protection with single dose in a primate challenge model and in a human ACE2 receptor transgenic mouse challenge model
   - ARCT-021 demonstrated robust protection with a single dose in mice depleted of B cells, whereas depletion of T lymphocytes yielded no protection following virus challenge, emphasizing the importance of ARCT-021 induced cellular immunity

4) **Manufacturing**:
   - Recently received $46.6M from Singapore EDB to support ARCT-021 stockpiling
   - Manufacturing of lyophilized ARCT-021 to support Phase 3 and initial commercial supply well on track
   - Stability studies for lyophilized ARCT-021 at -20°C, 2-8°C and room temperature storage conditions ongoing

5) **Agreements**:
   - Strategic, government, and country supply agreement discussions continue to progress

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

- Received approval from Health Canada to enroll subjects into Phase 1b study
- CTA filing for Phase 2 multiple dose study on track for Q2

ARCT-032, Therapeutic Candidate for Cystic Fibrosis

- Completed successful pre-IND interaction with FDA
- CTA filing on track for Q4

Acquisition of Exclusive License to mRNA Manufacturing Technology from Alexion Pharmaceuticals

- The technology supports Arcturus' highly efficient processes to manufacture high purity mRNA vaccine and therapeutic candidates at kilogram scale
- Extends the substantial intellectual property portfolio held by Arcturus

Financial results for the quarter and full year ended December 31, 2020

Revenues *in conjunction with strategic alliances and collaborations*: Arcturus' primary source of revenues is from license fees and collaboration payments received from research and development arrangements with our pharmaceutical and biotechnology partners.

On a quarterly basis, total revenue for the three months ended December 31, 2020 was $2.2 million and was relatively flat when compared to the $2.3 million of the quarter ended September 30, 2020.

On a yearly basis, reported revenues of $9.5 million during the year ended December 31, 2020, decreased from $20.8 million in the year ended December 31, 2019. The decline in collaboration revenues primarily relates to three factors: a $5.6 million decrease in reimbursements from CureVac associated with the OTC collaboration that ended in the third quarter of 2019, a decrease in one-time license revenue of $3.3 million from Synthetic Genomics that occurred in 2019, and lower activity with other collaboration partners.

Operating expenses*:* On a quarterly basis, total operating expenses for the three months ended December 31, 2020 were $33.3 million compared with $23.3 million for quarter ended September 30, 2020, and $13.8 million in same period of 2019. Approximately $8 million of the sequential increase in operating expenses during the quarter ended December 31, 2020 was due to the ramp in the Covid-19 program related expenses, which included additional personnel, manufacturing and clinical trial expenses. The current quarter operating expenses were partially offset by $2.7 million in funds awarded under the Singapore vaccine grants and by the Cystic Fibrosis Foundation.

On a yearly basis, total operating expenses were $81.1 million for the year-ended December 31, 2020 compared with $46.3 million for the year ended December 31, 2019. The current year operating expenses were partially offset by $15.2 million of funds earned under the Singapore vaccine grants and funds awarded by the Cystic Fibrosis Foundation. The increase in net expenditures for the year ended December 31, 2020 as compared to the prior year was due primarily to the increased activity in clinical and manufacturing expenditures related to the Company's Covid-19 and OTC programs as well as increased personnel costs and other facility costs related to the organizational growth of the Company.

Net loss: For the three months ended December 31, 2020 Arcturus reported a net loss of approximately $31.1 million, or ($1.25) per basic and diluted share, compared with a net loss in the three months ended September 30, 2020 of $21.0 million, or ($0.92) per basic and diluted share, and three months ended December 31, 2019 of $11.0 million, or ($0.76) per basic and diluted share.

For the year ended December 31, 2020, net loss was approximately $72.1 million, or ($3.55) per basic and diluted share, compared with a net loss for the year ended 2019 of $26.0 million, or ($2.15) per basic and diluted share.

Cash and Cash Equivalents: The Company's cash balance was $463.0 million as of December 31, 2020, compared to cash and cash equivalents of $301.1 million on September 30, 2020. The increase in cash and cash equivalents compared to the prior year is primarily due to the receipt of approximately $162 million in net proceeds from our December 2020 public offering. Subsequent to the end of the quarter, in January 2021 the Company received $46.6 million in funds under a manufacturing loan from Singapore EDB for our Covid-19 vaccine program. Based on our current pipeline, the Company's cash position is expected to be sufficient to support operations for more than two years.

Monday, March 1 @ 4:30 p.m. ET

Domestic:	877-407-0784
International:	201-689-8560
Conference ID:	13716298
Webcast:	http://public.viavid.com/index.php?id=143486

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 likelihood of success, and the efficacy or safety, of the Company's pipeline, including ARCT-021, ARCT-810 or ARCT-032, 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 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 or to manufacture and scale up manufacturing of any other product or substance, the likelihood that a patent will issue from any patent application, the results of advancements in the Company's manufacturing methods and technologies, including purification and lyophilization, 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, 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.

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ARCTURUS THERAPEUTICS HOLDINGS INC. AND ITS SUBSIDIARIES

CONSOLIDATED BALANCE SHEETS

(in thousands, except par value information)

	As of December 31,	
	2020	2019
Assets		
Current assets:		
Cash and cash equivalents	$ 462,895	$ 71,353
Accounts receivable	2,125	2,179
Prepaid expenses and other current assets	2,769	758
Total current assets	467,789	74,290
Property and equipment, net	3,378	2,349
Operating lease right-of-use asset, net	5,182	5,134
Equity-method investment	—	263
Non-current restricted cash	107	107
Total assets	$ 476,456	$ 82,143
Liabilities and stockholders' equity		
Current liabilities:		
Accounts payable	$ 10,774	$ 5,793
Accrued liabilities	20,639	7,134
Deferred revenue	18,108	8,397
Total current liabilities	49,521	21,324
Deferred revenue, net of current portion	12,512	15,182
Long-term debt, net of current portion	13,845	14,995
Operating lease liability, net of current portion	4,025	4,850
Total liabilities	79,903	56,351
Stockholders' equity:		
Common stock: $0.001 par value; 60,000 shares authorized and 26,192 shares issued and outstanding at December 31, 2020; 30,000 shares authorized and 15,138 shares issued and outstanding at December 31, 2019	26	15
Additional paid-in capital	540,343	97,445
Accumulated deficit	(143,816)	(71,668)
Total stockholders' equity	396,553	25,792
Total liabilities and stockholders' equity	$ 476,456	$ 82,143

ARCTURUS THERAPEUTICS HOLDINGS INC. AND ITS SUBSIDIARIES

CONSOLIDATED STATEMENTS OF OPERATIONS AND COMPREHENSIVE LOSS

(in thousands, except per share data)

	Year Ended December 31,		
	2020	2019	2018
Collaboration revenue	$ 9,539	$ 20,789	$ 15,753
Operating expenses:			
Research and development, net	57,846	33,640	16,982
General and administrative	23,217	12,662	20,582
Total operating expenses	81,063	46,302	37,564
Loss from operations	(71,524)	(25,513)	(21,811)
Loss from equity-method investment	(263)	(32)	(302)
Finance (expense) income, net	(361)	(446)	328
Net loss	(72,148)	(25,991)	(21,785)
Net loss per share, basic and diluted	$ (3.55)	$ (2.15)	$ (2.16)
Weighted-average shares outstanding, basic and diluted	20,305	12,069	10,069
Comprehensive loss:			
Net loss	$ (72,148)	$ (25,991)	$ (21,785)
Unrealized gain on short-term investments	—	—	3
Comprehensive loss	$ (72,148)	$ (25,991)	$ (21,782)

	Fourth Quarter 2019 (unaudited)	First Quarter 2020 (unaudited)	Second Quarter 2020 (unaudited)	Third Quarter 2020 (unaudited)	Fourth Quarter 2020 (unaudited)
Collaboration revenue	$ 2,968	$ 2,646	$ 2,322	$ 2,333	$ 2,238
Research and development expenses, net	11,994	7,917	7,944	17,699	24,286
General and administrative expenses	1,791	4,191	4,420	5,572	9,034
Loss from operations	(10,817)	(9,462)	(10,042)	(20,938)	(31,082)
Net loss	(10,989)	(9,777)	(10,263)	(21,004)	(31,104)
Net loss per share, basic and diluted	$ (0.76)	$ (0.67)	$ (0.55)	$ (0.92)	$ (1.25)
Weighted average shares outstanding, basic and diluted	14,505	14,521	18,794	22,938	24,886