



Arcturus Therapeutics Provides Corporate Update and Reports Fourth Quarter and Year End 2018 Financial Results

March 18, 2019

SAN DIEGO, March 18, 2019 (GLOBE NEWSWIRE) -- Arcturus Therapeutics Ltd. (NASDAQ: ARCT), a leading messenger RNA medicines company focused on the discovery, development and commercialization of therapeutics towards rare diseases, today reported its financial results for the quarter and year ended December 31, 2018, and provided a corporate update.

"We are excited about the progress Arcturus is making in advancing our portfolio of RNA medicines which we are developing for liver disease, cystic fibrosis, genetic diseases and vaccine applications," said Joseph Payne, President & CEO of Arcturus Therapeutics. "Our most advanced program, ARCT-810, to treat ornithine transcarbamylase (OTC) deficiency, is currently being assessed in IND-enabling studies with the goal of filing an IND in the fourth quarter this year. Over the course of 2019, we look forward to reporting on further developments in our pipeline and platform technologies."

Recent Highlights

Advanced Flagship mRNA Program for Ornithine Transcarbamylase (OTC) Deficiency

- Nominated ARCT-810 as a clinical development candidate to treat OTC deficiency in January 2019.
- Reassumed 100% global rights for ARCT-810 in February 2019.
- Regulatory agencies in the U.S. and Europe have been informed of our CMC specifications along with our non-clinical and clinical design plans. We have received guidance in these regards.
- Arcturus remains on track to file an IND for ARCT-810 with the U.S. Food and Drug Administration (FDA) in 4Q 2019.
- Presented new data on our Ornithine Transcarbamylase (OTC) Deficiency program at the Leerink 8th Annual Global Healthcare Conference during Feb 27th-Mar 1st
 - Efficacy Data – 0.1 mg/kg to 1 mg/kg in mouse disease model.
 - Safety Data – Dosing up to 7 mg/kg in mice.
- Key Opinion Leader (KOL) call in November 2018 on current treatment options for OTC Deficiency
 - Call featured a presentation by KOL Dr. Annette Feigenbaum, MBChB, FRCP, DABMG, from the University of California - San Diego, who discussed the disease pathogenesis, manifestation, natural history and current treatment options for ornithine transcarbamylase OTC deficiency.
 - Dr. Pad Chivukula, Chief Scientific Officer of Arcturus Therapeutics, provided an overview of the company's ongoing LUNAR-OTC program.

Advanced Platform Technologies

- LUNAR® Delivery
 - Safety Data presented at the Leerink 8th Annual Global Healthcare Conference during Feb 27th-Mar 1st.
 - Highlighted multi-dose primate data (8 weekly doses @ 3 mg/kg, 24 mg/kg total in 50 days).
- Arcturus mRNA Manufacturing capabilities have matured significantly
 - 30g non-GMP can be produced in less than a week.
 - Proprietary purification process identified to reduce double-stranded RNA impurities.

Senior Management Appointment

- Appointed Andrew Sassine as Chief Financial Officer.

Expanded Operations

- New San Diego facility is over 24,700 square feet and is designed to enable Arcturus to advance its pipeline and platform technologies.

Financial Results for the Quarter and Year Ended December 31, 2018

Revenues in conjunction with strategic alliances and collaborations The Company enters into research and development arrangements with pharmaceutical and biotechnology partners. For the fourth quarter of 2018, Arcturus reported revenue of \$7.6 million, compared with \$2.0 million during fourth quarter of 2017.

For the year ended December 31, 2018, Arcturus reported revenue of \$15.8 million, compared with revenue of \$13.0 million during the year ended December 31, 2017.

Operating expenses Total operating expenses for the fourth quarter of the 2018 were \$8.3 million compared with \$7.0 million for the same period of 2017.

Total operating expenses for the year ended December 31, 2018 were \$37.6 million compared with \$23.5 million for the same period in 2017.

Net Loss. For the fourth quarter ended December 31, 2018, Arcturus reported a net loss of approximately \$1.0 million, or (\$0.10) per basic and diluted share, compared with a net loss for the fourth quarter of 2017 of \$5.3 million, or (\$0.86) per basic and diluted share.

For the year ended December 31, 2018, net loss was approximately \$21.8 million, or (\$2.16) per basic and diluted share, compared with a net loss for the year ended 2017 of \$10.9 million, or (\$3.53) per basic and diluted share.

Cash, cash equivalents, and investments totaled \$36.7 million as of December 31, 2018. Based on current projections, the Company's current cash position is expected to be sufficient to support operations through early first quarter of 2020.

About ARCT-810

ARCT-810, Arcturus' first development candidate, represents a novel approach to treat ornithine transcarbamylase deficiency (OTCD). ARCT-810 based on Arcturus mRNA design construct and proprietary manufacturing process. ARCT-810 also utilizes Arcturus' propriety lipid library and employs the Company's LUNAR[®] delivery platform to safely and effectively deliver OTC mRNA to hepatocytes. ARCT-810 is an mRNA replacement therapy designed to enable OTC-deficient patients to naturally produce healthy functional OTC enzyme in their own liver cells. Arcturus is currently on track to submit an Investigational New Drug Application (IND) to the FDA in the fourth quarter of 2019. ARCT-810 is advancing toward the clinic on the strength of preclinical proof-of-concept data, demonstrating that LUNAR technology can deliver mRNA to liver cells and results in expression of functional OTC protein in animal models. Replacing the deficient OTC protein restores the urea cycle pathway, resulting in reduced plasma ammonia and urinary orotate concentrations.

About Ornithine Transcarbamylase Deficiency (OTCD)

OTC deficiency is caused by mutations in the OTC gene which leads to a non-functional or deficient OTC enzyme. OTCD is the most common urea cycle disorder. Urea cycle disorders are a group of inherited metabolic disorders that make it difficult for afflicted patients to remove toxic waste products, as proteins are digested. OTC deficiency is a life-threatening genetic disease. OTC is a critical enzyme in the urea cycle, which takes place in liver cells, and converts ammonia to urea. This conversion does not occur properly in patients with OTC deficiency and ammonia accumulates in their blood, acting as a neurotoxin and liver toxin. This can cause severe symptoms including vomiting, headaches, coma and death. OTC deficiency is an inherited disease that can cause developmental problems, seizures and death in newborn babies. It is an X-linked disorder, so is more common in boys. Patients with less severe symptoms may present later in life, as adults. There is currently no cure for OTC deficiency, apart from liver transplant. However, this treatment comes with significant risk of complications such as organ rejection, and transplant recipients must take immunosuppressant drugs for the rest of their lives. Current standard of care for OTC patients is a low-protein diet and ammonia scavengers to try and prevent patients from accumulating ammonia. These treatments do not address the underlying cause of disease.

About Arcturus Therapeutics Ltd.

Founded in 2013 and based in San Diego, California, Arcturus Therapeutics Ltd. (NASDAQ: ARCT) is an RNA medicines company with enabling technologies – UNA Oligomer chemistry and LUNAR[®] lipid-mediated delivery. Arcturus' diverse pipeline of RNA therapeutics includes programs pursuing rare diseases, Hepatitis B, non-alcoholic steatohepatitis (NASH), cystic fibrosis, and vaccines. Arcturus' versatile RNA therapeutics platforms can be applied toward multiple types of RNA medicines including small interfering RNA, messenger RNA, replicon RNA, antisense RNA, microRNA and gene editing therapeutics. Arcturus owns LUNAR lipid-mediated delivery and Unlocked Nucleomonomer Agent (UNA) technology including UNA Oligomers, which are covered by its extensive patent portfolio (152 patents and patent applications, issued in the U.S., Europe, Japan, China and other countries). Arcturus' proprietary UNA technology can be used to target individual genes in the human genome, as well as viral genes, and other species for therapeutic purposes. Arcturus' commitment to the development of novel RNA therapeutics has led to partnerships with Janssen Pharmaceuticals, Inc., part of the Janssen Pharmaceutical Companies of Johnson & Johnson, Ultragenyx Pharmaceutical, Inc., Takeda Pharmaceutical Company Limited, Synthetic Genomics Inc. and the Cystic Fibrosis Foundation. For more information, visit www.Arcturusrx.com, the content of which is not incorporated herein by reference.

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 regarding strategy, future operations, collaborations, future financial position, prospects, plans and objectives of management, the likelihood of success of the Company's technology or potential development of any products, including statements relating to the status of the preclinical development program for ARCT-810 or the other clinical development programs of Arcturus, the date that an IND may be filed with the FDA, the potential for ARCT-810 or the other clinical development programs of Arcturus, current standards of care, and the Company's future cash position 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. Actual results and performance could differ materially from those projected in any forward-looking statements as a result of many factors, including without limitation, an inability to develop and market product candidates, inability to generate positive verifiable data, unexpected clinical results, unforeseen expenses and general market conditions that may prevent such achievement or performance. 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, 2018, filed with the SEC on March 18, 2019 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 LTD. AND ITS SUBSIDIARIES

CONSOLIDATED BALANCE SHEETS

(In U.S. dollars in thousands, except par value information)

	As of December 31,	
	2018	2017
Assets		
Current assets:		
Cash and cash equivalents	\$ 36,709	\$ 24,965
Restricted cash	—	166
Short-term investments	—	23,608
Accounts receivable, net	4,481	480
Prepaid expenses and other current assets	638	1,059
Intangible asset held for sale	—	590
Total current assets	41,828	50,868
Property and equipment, net	1,975	1,049
Equity method investment	288	—
Non-current restricted cash	107	107
Total assets	\$ 44,198	\$ 52,024
Liabilities and shareholders' equity		
Current liabilities:		
Accounts payable	\$ 2,398	\$ 1,790
Accrued liabilities	3,907	2,793
Deferred revenue	6,272	6,457
Total current liabilities	12,577	11,040
Deferred revenue, net of current portion	7,534	7,190
Long-term debt	9,911	—
Deferred rent	534	—
Total liabilities	30,556	18,230
Commitments and contingencies (Note 13)		
Shareholders' equity:		
Ordinary shares: 30,000 shares authorized, 10,762 issued, 10,719 outstanding and 43 held in treasury at December 31, 2018; NIS 0.07 par value; 30,000 shares authorized, 10,699 issued, 10,656 outstanding and 43 held in treasury at December 31, 2017;	214	212
Additional paid-in capital	58,302	56,674
Accumulated other comprehensive loss	—	(3)
Accumulated deficit	(44,874)	(23,089)
Total shareholders' equity	13,642	33,794
Total liabilities and shareholders' equity	\$ 44,198	\$ 52,024

ARCTURUS THERAPEUTICS LTD. AND ITS SUBSIDIARIES

CONSOLIDATED STATEMENTS OF OPERATIONS AND COMPREHENSIVE LOSS

U.S. dollars in thousands (except per share data)

	Year Ended December 31,	
	2018	2017
Collaboration revenue	\$ 15,753	\$ 12,998
Operating expenses:		

Research and development	16,982	15,918
General and administrative	20,582	7,572
Total operating expenses	<u>37,564</u>	<u>23,490</u>
Net loss from operations	(21,811)	(10,492)
Loss from equity-method investment	(302)	—
Finance income (expense), net	328	(409)
Net loss before taxes	(21,785)	(10,901)
Income tax expense	—	(1)
Net loss	<u>\$ (21,785)</u>	<u>\$ (10,902)</u>
Net loss per share, basic and diluted	<u>\$ (2.16)</u>	<u>\$ (3.53)</u>
Weighted-average shares outstanding, basic and diluted	<u>10,069</u>	<u>3,087</u>
Comprehensive loss:		
Net loss	\$ (21,785)	\$ (10,902)
Unrealized gain (loss) on short-term investments	3	(3)
Comprehensive loss	<u>\$ (21,782)</u>	<u>\$ (10,905)</u>

ARCTURUS THERAPEUTICS LTD. AND ITS SUBSIDIARIES

SELECTED FINANCIAL DATA

(in thousands, except per share and share data)	For the quarter ended				
	December 31, 2018	September 30, 2018	June 30, 2018	March 31, 2018	December 31, 2017
	(Unaudited)				
Revenue in conjunction with strategic alliances and collaborations	\$ 7,577	\$ 3,423	\$ 2,386	\$ 2,367	\$ 2,020
Research and development expenses, net	4,847	3,969	4,225	3,941	4,353
General and administrative expenses	3,441	3,810	8,233	5,098	2,634
Net loss from operations	(711)	(4,356)	(10,072)	(6,672)	(4,967)
Net loss	(1,011)	(4,253)	(9,950)	(6,571)	(5,280)
Net loss per share, basic and diluted	\$ (0.10)	\$ (0.42)	\$ (0.99)	\$ (0.66)	\$ (0.86)
Weighted average shares outstanding, basic and diluted	10,095,392	10,092,891	10,057,048	10,027,834	6,151,580
	As of				
	December 31, 2018	September 30, 2018	June 30, 2018	March 31, 2018	December 31, 2017
	(Unaudited)				
Working capital	\$ 29,251	\$ 20,801	\$ 23,429	\$ 37,383	\$ 39,828
Total assets	\$ 44,198	\$ 37,115	\$ 44,322	\$ 52,483	\$ 52,024
Shareholders' equity (deficit)	\$ 13,642	\$ 14,151	\$ 17,795	\$ 27,543	\$ 33,794



Source: Arcturus Therapeutics, Inc.