

Rubius Therapeutics Reports Third Quarter 2018 Financial Results and Operational Progress

November 13, 2018

On Track to Submit First IND for RTX-134 in Q1 2019

CAMBRIDGE, Mass., Nov. 13, 2018 (GLOBE NEWSWIRE) -- Rubius Therapeutics, Inc. (Nasdaq: RUBY), a biotechnology company developing an entirely new class of allogeneic cellular therapies, today reported third quarter 2018 financial results and operational progress.

"During the third quarter, we remained focused on advancing our first program, RTX-134, for the treatment of phenylketonuria. We are on track to file an Investigational New Drug application during the first quarter of 2019," said Pablo J. Cagnoni, M.D., chief executive officer of Rubius Therapeutics. "We are also continuing to advance our earlier pipeline, which we believe holds broad therapeutic potential across cancer, autoimmune disease and additional enzyme deficiencies. In order to successfully bring our medicines to patients, we are ensuring that we will have state-of-the-art manufacturing capabilities in place, as shown through the purchase, in July, of our 135,000-square foot manufacturing facility in Smithfield, RI."

Third quarter highlights include:

- On track to submit first IND for lead program, RTX-134, during the first quarter of 2019, and an additional three to four INDs during 2019 and 2020.
- Closed initial public offering (IPO) in July 2018, raising \$254.3 million in net proceeds
- Acquired and initiated renovations on manufacturing facility in Smithfield, RI; facility is expected to be operational by the end of 2020
- Continued to generate promising preclinical data in support of additional pipeline programs; data expected to be published during 2019
- Strengthened internal capabilities in discovery, platform development and manufacturing and grew the organization to 110 employees to predominantly support research and development (R&D) activities

Third Quarter Financial Results

Net loss for the third quarter of 2018 was \$26.4 million or \$0.42 per common share, compared to \$11.9 million or \$1.48 per common share in the third quarter of 2017.

In the third quarter of 2018, Rubius invested \$14.4 million in R&D related to its novel RED PLATFORM[®] and towards expanding and advancing its product pipeline, compared to \$6.1 million in the third quarter of 2017. The year-over-year increase was due to an additional \$2.9 million of costs incurred in preparation for the Phase 1/2a clinical trial for RTX-134, and \$3.6 million was associated with personnel costs and stock-based compensation driven by increases in R&D headcount to support Rubius' goal of delivering four to five IND's during 2019 and 2020.

G&A expenses were \$13.2 million during the third quarter of 2018, as compared to \$5.8 million for the third quarter of 2017. The higher costs were primarily driven by a \$4.6 million increase in stock-based compensation and a \$1.9 million increase in personnel costs and professional fees to support the Company's growth and to operate as a public company.

Nine Month Financial Results

Net loss for the first nine months of 2018 was \$62.0 million or \$2.33 per common share, compared to \$27.0 million or \$3.42 per common share in the first nine months of 2017.

In the nine months ended September 30, 2018, Rubius invested \$35.2 million in R&D related to its novel RED PLATFORM[®] and towards expanding and advancing its product pipeline, compared to \$14.6 million in the first nine months of 2017. The year-over-year increase was due to an increase of \$5.8 million of costs incurred in preparation for the Phase 1/2a clinical trial for RTX-134, and \$6.6 million in personnel costs and stock-based compensation driven by increases in R&D headcount to support Rubius' goal of delivering four to five IND's during 2019 and 2020.

G&A expenses were \$27.3 million during the first nine months of 2018, as compared to \$11.2 million for the same period in 2017. The higher costs were primarily driven by a \$8.7 million increase in stock-based compensation and a \$6.0 million increase in personnel costs and professional fees to support the Company's growth and to operate as a public company.

During the third quarter of 2018, the Company adopted new guidance for the accounting for stock-based payments to nonemployees, effective as of January 1, 2018. As a result of this adoption, previously reported amounts for the six months ended June 30, 2018 for R&D expenses and G&A expenses were reduced by \$0.7 million and \$8.0 million, respectively.

Cash Position

As of September 30, 2018, cash, cash equivalents and investments grew significantly to \$408.9 million as compared to \$104.3 million as of December 31, 2017, providing Rubius with a cash runway into 2021. The increase in cash reflects \$254.3 million of net proceeds from the company's IPO during the third quarter of 2018 and \$101.0 million of net proceeds received from its Series C preferred stock financing during the first quarter of 2018. The proceeds received from the financings were offset by \$38.6 million used in operations during the nine-month period and \$11.7 million of capital purchases, including \$8.0 million to acquire the manufacturing facility in Smithfield, Rhode Island.

About Phenylketonuria and RTX-134

Phenylketonuria (PKU) is an inherited, rare enzymatic disorder characterized by the body's inability to effectively metabolize the amino acid phenylalanine. The accumulation of phenylalanine in the blood causes damage to the central nervous system and a range of symptoms, including intellectual disability, delayed development and impaired cognitive function. RTX-134 is an allogeneic cellular therapy for the treatment of PKU, which expresses the enzyme phenylalanine ammonia lyase (PAL) inside the cell. In preclinical studies, phenylalanine was shown to diffuse into RTX-134, where PAL converted phenylalanine into ammonia and trans-cinnamic acid, metabolites that are cleared by the body. Compared to current therapeutic

interventions, RCT™ product candidates may have a longer and more sustained treatment duration given the 120-day half-life of red blood cells and may avoid immune-driven adverse events and reduction in efficacy, which result from antibody formation.

About Rubius Therapeutics

Rubius Therapeutics is a biopharmaceutical company developing a new class of medicines called Red Cell Therapeutics™. The Company's proprietary RED PLATFORM® was designed to genetically engineer and culture Red Cell Therapeutics™ that are selective, potent and ready-to-use allogeneic cellular therapies for the potential treatment of several diseases across multiple therapeutic areas. Rubius' initial focus is to advance RCT™ product candidates for the treatment of rare diseases, cancer and autoimmune diseases by leveraging three distinct therapeutic modalities — cellular shielding, potent cell-cell interaction and tolerance induction. For more information, visit www.rubiustx.com, or follow us on [Twitter](#) and [LinkedIn](#).

Forward Looking Statements

This press release contains forward-looking statements within the meaning of the Private Securities Litigation Reform Act of 1995, as amended, including, without limitation, statements regarding the renovation and operation of our manufacturing facility, the therapeutic potential of our Red Cell Therapeutics, including RTX-134 for the treatment of PKU, the timeline for us to file an IND, and our strategy, business plans and focus. The words "may," "will," "could," "would," "should," "expect," "plan," "anticipate," "intend," "believe," "estimate," "predict," "project," "potential," "continue," "target" and similar expressions are intended to identify forward-looking statements, although not all forward-looking statements contain these identifying words. Any forward-looking statements in this press release are based on management's current expectations and beliefs and are subject to a number of risks, uncertainties and important factors that may cause actual events or results to differ materially from those expressed or implied by any forward-looking statements contained in this press release, including, without limitation, those risks and uncertainties related to the development of our Red Cell Therapeutic product candidates and their therapeutic potential and other risks identified in our SEC filings, including our Quarterly Report on Form 10-Q for the quarter ended June 30, 2018, and subsequent filings with the SEC. We caution you not to place undue reliance on any forward-looking statements, which speak only as of the date they are made. We disclaim any obligation to publicly update or revise any such statements to reflect any change in expectations or in events, conditions or circumstances on which any such statements may be based, or that may affect the likelihood that actual results will differ from those set forth in the forward-looking statements. Any forward-looking statements contained in this press release represent our views only as of the date hereof and should not be relied upon as representing its views as of any subsequent date. We explicitly disclaim any obligation to update any forward-looking statements.

Rubius Therapeutics, Inc.

Condensed Consolidated Statement of Operations (in thousands, except share and per share data) (unaudited)

	For the three months ended September 30,		For the nine months ended September 30,	
	2018	2017	2018 ¹	2017
Revenue	\$ —	\$ —	\$ —	\$ —
Operating expenses:				
Research and development	14,363	6,126	35,230	14,628
General and administrative	13,191	5,849	27,311	11,163
Total operating expenses	27,554	11,975	62,541	25,791
Loss from operations	(27,554)	(11,975)	(62,541)	(25,791)
Other income (expense), net	1,192	56	529	(539)
Net loss	\$ (26,362)	\$ (11,919)	\$ (62,012)	\$ (26,330)
Accretion of Series A redeemable convertible preferred stock to redemption value	—	—	—	(656)
Net loss attributable to common stockholders	\$ (26,362)	\$ (11,919)	\$ (62,012)	\$ (26,986)
Net loss per share attributable to common stockholders, basic and diluted	\$ (0.42)	\$ (1.48)	\$ (2.33)	\$ (3.42)
Weighted average common shares outstanding, basic and diluted:	62,311,111	8,078,196	26,662,233	7,899,507

¹ During the third quarter of 2018, the Company adopted new guidance for the accounting for stock-based payments to nonemployees, effective as of January 1, 2018. As a result of this adoption, previously reported amounts for the six months ended June 30, 2018 for research and development expenses and general and administrative expenses were reduced by \$0.7 million and \$8.0 million, respectively.

Rubius Therapeutics, Inc.

Condensed Consolidated Balance Sheet Data (in thousands) (unaudited)

	September 30, 2018	December 31, 2017
Cash, cash equivalents and investments	\$ 408,874	\$ 104,288
Total assets	452,798	107,687
Total liabilities	41,791	11,584

Total stockholders' deficit and convertible preferred stock

411,007

96,103

Media Contact:

Dan Budwick

1AB, Inc.

+1 (973) 271-6085

dan@1abmedia.com

Investor Contact:

Lori Melançon

Vice President, Corporate Communications and Investor Relations

+1 (617) 949-5296

lori.melancon@rubiustx.com



Source: Rubius Therapeutics