



Rubius Therapeutics Reports Second Quarter 2019 Financial Results and Operational Progress

August 13, 2019

On Track to Report Initial Clinical Data from Phase 1b Clinical Trial of RTX-134 for Phenylketonuria by Year-End

CAMBRIDGE, Mass., Aug. 13, 2019 (GLOBE NEWSWIRE) -- Rubius Therapeutics, Inc. (Nasdaq:RUBY), a clinical-stage biopharmaceutical company that is genetically engineering red blood cells to create an entirely new class of cellular medicines, today reported second quarter 2019 financial results and provided an overview of operational progress.

"During the second quarter, we continued to advance our promising pipeline of Red Cell Therapeutics™ for the potential treatment of rare diseases, cancer and autoimmune diseases," said Pablo J. Cagnoni, M.D., chief executive officer of Rubius Therapeutics. "In addition, we have been working closely with our contract manufacturer to improve its ability to consistently produce product in 200-liter bioreactors. We expect to begin enrolling patients in the near term and remain on track to deliver initial clinical data by year-end. Finally, we continue to make great progress on the buildout of our new manufacturing facility in Smithfield, RI to provide GMP manufacturing for clinical supply."

Second Quarter Highlights and Upcoming Milestones

- Rubius is working closely with its contract manufacturer to improve its ability to consistently produce product in 200-liter bioreactors in order for Rubius to begin enrolling patients in its Phase 1b clinical trial for patients with phenylketonuria (PKU) in the near term.
- Rubius plans to report initial clinical data from the first patients enrolled in the trial by year-end, including:
 - Preliminary safety;
 - Longevity of RTX-134 cells in circulation; and
 - Proof-of-mechanism as measured by production of trans-cinnamic acid, the metabolite of phenylalanine when degraded by phenylalanine ammonia lyase, or PAL.
- The Company continued to strengthen its leadership by [appointing](#) Kris Elverum as senior vice president of business development and strategy to oversee corporate strategy and create and execute potential new business development opportunities.
- On June 5, 2019, Rubius [announced](#) the issuance of three patents from the U.S. Patent and Trademark Office related to its rare disease and oncology programs and platform.
 - Two of the issued patents cover composition of matter and methods of use for the Company's lead investigational cellular therapy, RTX-134, for the treatment of patients with PKU.
 - The third patent, the Company's first issued composition of matter patent in immuno-oncology, relates to human cells having a checkpoint inhibitor linked to the cell surface.
 - Today, in total, Rubius Therapeutics has 30 patent families and more than 130 pending patent applications worldwide.

Second Quarter Financial Results

Net loss for the second quarter of 2019 was \$39.4 million or \$0.50 per common share, compared to \$21.2 million or \$2.43 per common share in the second quarter of 2018.

In the second quarter of 2019, Rubius invested \$27.5 million in research and development (R&D) related to its novel RED PLATFORM® and towards expanding and advancing its product pipeline, as compared to \$11.4 million in the second quarter of 2018. This year-over-year increase was driven primarily by \$7.6 million in incremental R&D program spending in preparation for the Company's Phase 1b clinical trial for RTX-134 and towards preclinical activities for Rubius' lead oncology programs, including RTX-240. In addition, \$7.4 million in incremental R&D spending was driven by increased R&D headcount, including technical operations, a move into larger facilities and purchasing lab supplies to support Rubius' goal of delivering four to five INDs across 2019 and 2020. R&D stock-based compensation also increased by \$1.9 million.

G&A expenses were \$13.8 million during the second quarter of 2019, as compared to \$9.0 million for the second quarter of 2018. The higher costs were primarily driven by a \$1.5 million increase in personnel and facility costs due to increased headcount in the general and administrative function, as well as increases in professional fees and infrastructure costs to support the Company's growth. G&A stock-based compensation also increased by \$2.8 million.

Six Month Financial Results

Net loss for the first six months of 2019 was \$72.0 million or \$0.92 per common share, compared to \$35.7 million or \$4.17 per common share in the first six months of 2018.

In the six months ended June 30, 2019, Rubius invested \$48.4 million in R&D related to its novel RED PLATFORM[®] and towards expanding and advancing its product pipeline, as compared to \$20.9 million in the first six months of 2018. This year-over-year increase was largely due to an additional \$14.9 million in R&D personnel, including technical operations, facilities and lab supplies to support the Company's pipeline expansion in 2019 and 2020 and \$11.2 million in R&D program spending, including preparing for the RTX-134 Phase 1b clinical trial and preclinical activities for Rubius' lead oncology programs, including RTX-240. R&D stock-based compensation also increased by \$3.6 million.

G&A expenses were \$27.3 million during the first six months of 2019, as compared to \$14.1 million for the same period in 2018. The higher costs were primarily driven by a \$7.9 million increase in stock-based compensation and a \$3.7 million increase in personnel costs and facility costs due to increased headcount in our general and administrative function, as well as increases in professional fees and infrastructure costs to support the Company's growth.

Cash Position

As of June 30, 2019, cash, cash equivalents and investments were \$362.3 million as compared to \$404.1 million as of December 31, 2018, providing Rubius with a cash runway into 2021. During the year, the Company used \$52.7 million of cash to fund operations and \$17.4 million to fund capital expenditures, including work related to the buildout of Rubius' manufacturing facility. In addition, during the quarter, the Company drew down a second tranche of \$25.0 million from its \$75.0 million loan agreement with Solar Capital, which leaves a third tranche of \$25.0 million that can be drawn through June 2020, subject to the satisfaction of certain financial covenants.

About Rubius Therapeutics

Rubius Therapeutics is a clinical-stage 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 off-the-shelf 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 planned timing, recruitment, enrollment and results for our preclinical and clinical activities, including the Phase 1b clinical trial for RTX-134 for the treatment of PKU, our ability to further develop our RCT product candidates, our manufacturing process, our expectations regarding the therapeutic potential of our RCTs, our expectations regarding new members of our leadership team 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 RCT 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, 2019, 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 June 30,		For the six months ended June 30,	
	2019	2018	2019	2018
Revenue	\$	\$	\$	\$
Operating expenses:				
Research and development	27,518	11,361	48,389	20,867
General and administrative	13,767	9,023	27,302	14,120
Total operating expenses	41,285	20,384	75,691	34,987
Loss from operations	(41,285)	(20,384)	(75,691)	(34,987)
Other income (expense), net	1,895	(855)	3,720	(663)
Net loss	\$ (39,390)	\$ (21,239)	\$ (71,971)	\$ (35,650)
Net loss per share, basic and diluted	\$ (0.50)	\$ (2.43)	\$ (0.92)	\$ (4.17)
Weighted average common shares outstanding, basic and diluted:	78,396,714	8,727,392	77,972,757	8,542,362

Rubius Therapeutics, Inc.
Condensed Consolidated Balance Sheet Data
(in thousands)
(unaudited)

	June 30, 2019	December 31, 2018
Cash, cash equivalents and investments	\$ 362,330	\$ 404,051
Total assets	438,032	479,109
Total liabilities	94,388	86,101
Total stockholders' equity and convertible preferred stock	343,644	393,008

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