Rubius Therapeutics Reports Fourth Quarter and Full-Year 2018 Financial Results with Business Updates

March 28, 2019

Investigational New Drug Application Cleared for First-Ever Red Cell Therapeutic™, RTX-134, for Treatment of Phenylketonuria

Data from Company's Oncology Pipeline to be Presented at AACR 2019 Annual Meeting

CAMBRIDGE, Mass., March 28, 2019 (GLOBE NEWSWIRE) -- Rubius Therapeutics, Inc. (Nasdaq:RUBY), a clinical-stage biopharmaceutical company that is generating red blood cells and bioengineering them into an entirely new class of cellular medicines, today provided a business update and reported fourth quarter and full-year 2018 financial results.

"2018 was a transformative year for Rubius as we transitioned from a private, preclinical company into a public company on the brink of entering the clinic with our first program, RTX-134, for the treatment of patients with phenylketonuria," said Pablo J. Cagnoni, M.D., chief executive officer. "With our first Investigational New Drug application for RTX-134 now cleared by the U.S. Food and Drug Administration, we plan to begin treating patients in our Phase 1b trial during the second quarter with initial clinical data expected during the second half of the year. We will also be presenting preclinical data at the AACR 2019 Annual Meeting, supporting our growing oncology pipeline. We stand well capitalized and well positioned to achieve our goal of filing four to five IND's by the end of 2020, as we work to advance this new class of cellular medicines on behalf of patients."

Recent Business Highlights and Anticipated Upcoming Milestones

- In March 2019, Rubius announced that the U.S. FDA cleared the Company's Investigational New Drug (IND) application for RTX-134, an allogeneic, off-the-shelf cellular therapy for the potential treatment of patients with phenylketonuria (PKU), an inherited metabolic disorder that is characterized by the body's inability to metabolize the essential dietary amino acid, phenylalanine.
  - Rubius expects to begin treating patients in a Phase 1b clinical trial in the second quarter of 2019 and plans to report initial clinical data from the trial during the second half of 2019.
  - The primary objectives of the Phase 1b study are to evaluate preliminary safety, longevity of the RTX-134 cells in circulation, to obtain proof-of-mechanism as measured by production of trans-cinnamic acid (the byproduct of PAL) and select a preliminary dose and schedule.
- At this year's American Association for Cancer Research (AACR) Annual Meeting, being held March 29-April 3, Rubius will present preclinical data from its oncology pipeline, including its lead oncology candidates RTX-240 (formerly RTX-212) and RTX-224 for the treatment of solid tumors and RTX-aAPC (HPV+), an artificial antigen presenting cell, for the treatment of HPV+ tumors, including head and neck cancers.
- Rubius strengthened its leadership by appointingNatalie Holles to its board of directors and Greg Whitehead as senior vice president and chief quality officer.
- The Company plans to continue investing in its pipeline and expects to file its first oncology IND for RTX-240 for the treatment of solid tumors by early 2020, with two to three additional IND filings following in 2020.
- Rubius intends to continue renovating and hiring key personnel for its Smithfield, RI manufacturing facility with the goal of being operational in 1000L bioreactors by the end of 2020.

Successful Execution on Key Priorities in 2018

- Purchased 135,000 sq. ft. manufacturing facility in Smithfield, RI and initiated renovations
- Scaled manufacturing of RCTs from 50L to 200L bioreactors
- Transferred RTX-134 manufacturing process to contract manufacturing organization
- Executed three successful financings, including a $101.0 million crossover round, a $254.3 million initial public offering after expenses, and a recent debt financing of up to $75.0 million, which further extends cash runway into 2021
- Increased RCT storage stability from 28 days to 42 days with additional studies ongoing to explore extending RCT storage stability even further
- Continued to strengthen the board of directors, build a leading scientific team and attract experienced leadership to deliver against Rubius' objectives
- Strengthened internal capabilities in discovery, platform development, technical operations and manufacturing

Fourth Quarter Financial Results

Net loss for the fourth quarter of 2018 was $27.2 million or $0.35 per common share, compared to $17.5 million or $2.07 per common share in the fourth quarter of 2017.

In the fourth quarter of 2018, Rubius invested $16.5 million in research and development (R&D) related to its novel RED PLATFORM® and towards expanding and advancing its product pipeline, compared to $6.6 million in the fourth quarter of 2017. The year-over-year increase was due to an additional $2.4 million of costs incurred in preparation for the Phase 1b clinical trial for RTX-134, and $6.7 million was associated with personnel costs, stock-based compensation, facility and laboratory costs driven by increases in R&D headcount and expanded research activities to support Rubius' goal of delivering four to five IND's during 2019 and 2020.

G&A expenses were $12.6 million during the fourth quarter of 2018, as compared to $10.9 million for the fourth quarter of 2017. The higher costs were primarily driven by increases in professional fees and infrastructure costs to support the Company's growth and to operate as a public company.

Full Year Financial Results

Net loss for the full year 2018 was $89.2 million or $2.27 per common share, compared to $44.5 million or $5.55 per common share for the full year 2017.

For the full year 2018, Rubius invested $51.8 million in R&D related to its novel RED PLATFORM® and towards expanding and advancing its product pipeline, compared to $21.2 million for the full year 2017. The year-over-year increase was primarily driven by an increase of $8.3 million of costs incurred in preparation for the Phase 1b clinical trial for RTX-134, and $19.1 million in personnel costs, stock-based compensation, facility and laboratory costs driven by increases in R&D headcount and expanded research activities to support Rubius' goal of delivering four to five IND's during 2019 and 2020.

G&A expenses were $39.9 million during the twelve months of 2018, as compared to $22.0 million for the same period in 2017. The higher costs were primarily driven by a $7.6 million increase in stock-based compensation and $10.3 million increase in personnel costs, facility and professional fees to support the Company's growth and to operate as a public company.

Cash Flow Highlights

As of December 31, 2018, cash, cash equivalents and investments were $404.1 million as compared to $104.3 million as of December 31, 2017, providing Rubius with a cash runway into 2021. The year-end cash balance reflects $101.0 million of net proceeds received from its Series C preferred stock financing during the first quarter of 2018, $254.3 million of net proceeds from the Company's initial public offering during the third quarter of 2018, and, in the fourth quarter, $25.0 million from a debt financing. During the fourth quarter, the Company used $19.7 million of cash in operations and used $3.3 million for capital purchases. During the full year, the Company used $58.3 million of cash in operations and $15.0 million for capital purchases, including $8.0 million to acquire the manufacturing facility in Smithfield, Rhode Island.

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, 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 fund the further development of our Red Cell Therapeutic product candidates, statements regarding the renovation and operation of our manufacturing facility, our expectations regarding the therapeutic potential of our Red Cell Therapeutics, including RTX-134 for the treatment of PKU, the timelines for us to file additional INDs, 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 Annual Report on Form 10-K for the year ended December 31, 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 December 31,	For the year ended December 31,

	2018	2017	2018	2017
Revenue	$ -	$ -	$ -	$ -
Operating expenses:				
Research and development	16,539	6,598	51,769	21,226
General and administrative	12,583	10,875	39,894	22,038
Total operating expenses	29,122	17,473	91,663	43,264
Loss from operations	(29,122)	(17,473)	(91,663)	(43,264)
Other income (expense), net	1,939	(44)	2,468	(583)
Net loss	$ (27,183)	$ (17,517)	$ (89,195)	$ (43,847)
Accretion of Series A redeemable convertible preferred stock to redemption value	-	-	-	(656)
Net loss attributable to common stockholders	$ (27,183)	$ (17,517)	$ (89,195)	$ (44,503)
Net loss per share attributable to common stockholders, basic and diluted	$ (0.35)	$ (2.07)	$ (2.27)	$ (5.55)
Weighted average common shares outstanding, basic and diluted:	76,747,827	8,478,687	39,285,468	8,023,785

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

	December 31, 2018	December 31, 2017
Cash, cash equivalents and investments	$ 404,051	$ 104,288
Total assets	479,109	107,687
Total liabilities	86,101	11,584
Total stockholders' deficit and convertible preferred stock	393,008	96,103

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Source: Rubius Therapeutics