



Rubius Therapeutics Reports First Quarter 2019 Financial Results and Operational Progress

May 15, 2019

Enrollment for Phase 1b Trial of RTX-134 for Phenylketonuria on Track for Second Quarter 2019; Initial Clinical Data Anticipated in Second Half 2019

Company Announces Transition of President Torben Straight Nissen, Ph.D.

CAMBRIDGE, Mass., May 15, 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 reported first quarter 2019 financial results and provided an overview of operational progress. In addition, Rubius announced that company president, Torben Straight Nissen, Ph.D., will be leaving the Company at the end of July 2019, to join Flagship Pioneering where he has held a venture partner role since joining Rubius in 2016.

"Rubius has made significant progress this year, including the clearance of our first Investigational New Drug application for RTX-134 for the treatment of patients with phenylketonuria," said Pablo J. Cagnoni, M.D., chief executive officer of Rubius Therapeutics. "We plan to begin enrolling patients in the Phase 1b trial during the second quarter with initial clinical data expected during the second half of the year. We presented important preclinical data from our emerging oncology portfolio at AACR, demonstrating promising anti-tumor activity with no observed toxicities for our lead product candidates, RTX-240 and RTX-224, for the treatment of solid tumors and RTX-aAPC for the treatment of HPV-positive tumors. The year is off to a great start as we continue to advance our Red Cell Therapeutics towards our ultimate goal of treating patients who are underserved by available therapies."

"We are grateful to Torben for all his contributions towards building a world-class organization and for remaining with Rubius since I joined the company to continue supporting the scale-up of our manufacturing capabilities," continued Dr. Cagnoni. "During his tenure, Torben also took part in executing key financings, made important leadership hires and helped drive the formation and advancement of our portfolio of programs that target rare diseases, cancer and autoimmune diseases. We wish him the best going forward."

"I am very proud of the hard work, dedication and innovative spirit of the Rubius team that has led the company to where it is today," said Dr. Straight Nissen. "I believe Rubius' Red Cell Therapeutics approach has the potential to transform the future of cellular medicine, and I look forward to following the company as they work towards bringing these important therapies to patients. I wish all my talented colleagues at Rubius continued success as I transition to focus on earlier stage platform companies within the Flagship Pioneering ecosystem."

Dr. Straight Nissen will continue to provide consulting services to Rubius into 2020. With his departure, Spencer Fisk, senior vice president of technical operations, and Thomas Wickham, Ph.D., senior vice president of discovery, will report directly to Dr. Cagnoni.

First Quarter Program Highlights and Updates

- In March 2019, Rubius [received clearance](#) from the U.S. Food and Drug Administration (FDA) of the Company's Investigational New Drug (IND) application for RTX-134. RTX-134 is an allogeneic, off-the-shelf cellular therapy for the potential treatment of patients with phenylketonuria (PKU), a rare, inherited metabolic disorder that is characterized by the body's inability to metabolize the essential dietary amino acid, phenylalanine.
 - The Company expects to enroll up to 10 adult patients with PKU in the Phase 1b trial, with enrollment expected to begin during the second quarter of 2019 and initial clinical data anticipated in the second half of 2019, including:
 - Preliminary safety;
 - Longevity of RTX-134 cells in circulation;
 - Proof-of-mechanism as measured by production of trans-cinnamic acid, the metabolite of phenylalanine when degraded by phenylalanine ammonia lyase (PAL); and
 - Selection of a preliminary dose and schedule.
- On April 2, 2019, Rubius [presented preclinical data](#) from its emerging oncology pipeline at the American Association for Cancer Research (AACR) Annual Meeting.
 - [RTX-240 \(4-1BBL and 1L-15TP\)](#) and [RTX-224 \(4-1BBL and IL-12\)](#) are allogeneic cellular therapy product candidates that are engineered to replicate the human immune system by stimulating the adaptive and innate immune systems to induce an immune response.
 - RTX-240 and RTX-224 each demonstrated potent activation of the immune system, anti-tumor activity and a potentially broad therapeutic window when compared to agonists antibodies or recombinant cytokines.
 - Additionally, RTX-224 in combination with an anti-PD-1 antibody demonstrated substantial tumor shrinkage in a difficult-to-treat MC38 colon cancer model.
 - [Red Cell Therapeutic \(RCT™\) artificial antigen presenting cells](#) or RTX-aAPCs, are engineered to induce a tumor-

specific response by expanding tumor-specific T cells against a target antigen.

- RTX-aAPCs activated and significantly expanded antigen-specific T cells to promote substantial tumor regressions, leading to increased survival with no observed toxicity, in an OVA-positive murine model.
- The Company continued to strengthen its leadership by [appointing](#) Natalie Holles to its board of directors and Greg Whitehead as senior vice president and chief quality officer, while bolstering internal capabilities in discovery, platform development and technical operations.
- Rubius remains on track to submit a total of four to five INDs across 2019 and 2020, while continuing to progress additional pipeline programs in rare diseases, cancer and autoimmune diseases.

First Quarter Financial Results

Net loss for the first quarter of 2019 was \$32.6 million or \$0.42 per common share, compared to \$14.4 million or \$1.72 per common share in the first quarter of 2018.

In the first quarter of 2019, Rubius invested \$20.9 million in research and development (R&D) related to its novel RED PLATFORM[®] and towards expanding and advancing its product pipeline, compared to \$9.5 million in the first quarter of 2018. The year-over-year increase was due to an additional \$3.6 million of costs incurred in preparation for the Phase 1b clinical trial for RTX-134, and \$9.3 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 across 2019 and 2020. These increases were offset by a decrease in external manufacturing and research costs that were not related to programs.

G&A expenses were \$13.5 million during the first quarter of 2019, as compared to \$5.1 million for the first quarter of 2018. The higher costs were primarily driven by a \$7.4 million increase in personnel costs, stock-based compensation 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 and to operate as a public company.

Cash Position

As of March 31, 2019, cash, cash equivalents and investments were \$378.9 million as compared to \$404.1 million as of December 31, 2018, providing Rubius with a cash runway into 2021. During the quarter, the Company used \$22.9 million of cash to fund operations and \$4.2 million to fund capital expenditures.

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 further develop our RCT product candidates, our expectations regarding the therapeutic potential of our RCTs, including RTX-240 and RTX-224 for the treatment of solid tumors, RTX-aAPC for the treatment of HPV-positive tumors and 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 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 March 31, 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 March 31,

	<u>2019</u>	<u>2018</u>
Revenue	\$	\$
Operating expenses:		
Research and development	20,871	9,506
General and administrative	13,535	5,097
Total operating expenses	<u>34,406</u>	<u>14,603</u>
Loss from operations	(34,406)	(14,603)
Other income (expense), net	1,825	192
Net loss	<u>\$ (32,581)</u>	<u>\$ (14,411)</u>
Net loss per share, basic and diluted	<u>\$ (0.42)</u>	<u>\$ (1.72)</u>
Weighted average common shares outstanding, basic and diluted:	<u>77,544,089</u>	<u>8,355,276</u>

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

	<u>March 31,</u> <u>2019</u>	<u>December 31,</u> <u>2018</u>
Cash, cash equivalents and investments	\$ 378,913	\$ 404,051
Total assets	441,470	479,109
Total liabilities	69,834	86,101
Total stockholders' equity and convertible preferred stock	371,636	393,008

Contacts:

Lori Melançon
Vice President, Corporate Communications and Investor Relations
+1 (617) 949-5296
lori.melancon@rubiusrx.com

Media Contact:

Dan Budwick
1AB
+1 (973) 271-6085
dan@1abmedia.com



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