Rubius Therapeutics Reports Fourth Quarter and Full Year 2020 Financial Results and Provides Business Update

February 23, 2021

Reported Initial Key Takeaways in January 2021 Showing RTX-240 Stimulates Innate and Adaptive Immunity; Additional Clinical Results to be Presented in Early 2021

Escalated the Dose in RTX-240 Phase 1 Clinical Trial in Relapsed/Refractory Acute Myeloid Leukemia

Screening Patients in RTX-321 Phase 1 Clinical Trial in Advanced Human Papillomavirus 16-Positive Cancers

CAMBRIDGE Mass., Feb. 23, 2021 (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 called Red Cell Therapeutics™, today reported fourth quarter and full year 2020 financial results and provided a business update.

“Last year was one of strong execution for Rubius Therapeutics as we advanced our clinical trials in oncology and strengthened our in-house manufacturing capabilities,” said Pablo J. Cagnoni, M.D., president and chief executive officer of Rubius Therapeutics. “2021 is set to be an important year in which we plan to further advance our programs and report the clinical results from the Phase 1 trial of RTX-240 in advanced solid tumors early in the year. We are encouraged by the initial key clinical takeaways we presented in January, showing the ability of RTX-240 to stimulate innate and adaptive immune responses in advanced solid tumors. We are continuing to enroll patients in our trials for RTX-240 in relapsed/refractory acute myeloid leukemia and solid tumors and screen patients for the RTX-321 trial in advanced HPV 16-positive cancers.”

Fourth Quarter and Full Year Highlights

RTX-240 Phase 1/2 Clinical Program for Advanced Solid Tumors or Relapsed/Refractory Acute Myeloid Leukemia (AML)

- Escalated the dose and continue to enroll patients in the relapsed/refractory AML arm of the Phase 1 RTX-240 clinical trial
- At the J.P. Morgan Healthcare Conference in January 2021, the Company presented key takeaways from 5 cohorts (n=14) of the Phase 1/2 RTX-240 solid tumor clinical trial, showing activation and expansion of both NK and T cells, indicating the ability of RTX-240 to stimulate innate and adaptive immune responses. The key takeaways from the initial data were:
  - No treatment-related Grade 3 or Grade 4 adverse events and no dose limiting toxicities observed (n=14)
  - All patients showed activation of NK or T cells or both cell types (n=14)
  - In the majority of patients (n=8), all of the following were observed across dose levels:
    - Activation of NK cells, activation of T cells, expansion of NK cells and expansion of T cells
- As more patients are enrolled and data mature, the Company expects to disclose additional clinical results in early 2021 and plans to present the data at a medical conference

By showing that RTX-240 stimulates innate and adaptive immune responses, Rubius Therapeutics believes that the full data set from the Phase 1 clinical trial will validate its RED PLATFORM® and potentially de-risk clinical development of its oncology pipeline, in particular for RTX-321. RTX-321 has an encouraging preclinical data package and expresses combinations of agonists on the cell surface, similar to RTX-240.

RTX-321 Artificial Antigen-Presenting Cell (aAPC) Development Program for Human Papillomavirus (HPV) 16-Positive Cancers

- Phase 1 clinical trial of RTX-321 is screening for HLA-A*02:01-positive patients with advanced HPV 16-positive cancers, including cervical cancer, head and neck cancer and anal cancer

Achievements from Rubius Fully Owned Manufacturing Site

- Continue to provide consistent cGMP supply for the two Phase 1 arms in the ongoing RTX-240 trial and Phase 1 RTX-321 clinical trial
- Achieved increases in productivity and liquid in-vial shelf life for RTX-240
- Continously met red blood cell identity and target product profile criteria for clinical supply for RTX-240
- Introduced frozen drug substance for the first time as part of the IND application for RTX-321, resulting in a truly off-the-shelf cellular therapy with a potential shelf life of up to several years

Preclinical Data 2020 Summary
The Company presented preclinical oncology data for RTX-240 and RTX-321 at the following conferences:

- Society for Immunotherapy of Cancer (SITC) Annual Meeting;
- Federation of Clinical Immunology Societies (FOCIS) Virtual Annual Meeting;
- American Association for Cancer Research (AACR) Tumor Immunology Conference; and
- American Society of Gene & Cell Therapy 23rd Annual Meeting

Anticipated 2021 Catalysts and Operational Objectives

- Present additional clinical data from the Phase 1 RTX-240 solid tumor trial in early 2021
- Continue to enroll patients in the second Phase 1 arm of the RTX-240 clinical trial in patients with relapsed/refractory AML
- Dose the first patient in the RTX-321 clinical trial in HPV 16-positive tumors
- Continue to produce cGMP material for the RTX-240 and RTX-321 clinical trials
- Present an integrated clinical program for RTX-240, including plans for expansion cohorts, and plans for the Company's oncology pipeline

About RTX-240
RTX-240, Rubius Therapeutics' lead oncology program, is an allogeneic, off-the-shelf cellular therapy product candidate that is engineered to simultaneously present hundreds of thousands of copies of the costimulatory molecule 4-1BB ligand (4-1BBL) and IL-15TP (trans-presentation of IL-15 on IL-15Rα) in their native forms. RTX-240 is designed to broadly stimulate the immune system by activating and expanding both NK and memory T cells to generate a potent anti-tumor response.

About RTX-321
RTX-321, the Company's second oncology program, is an allogeneic, off-the-shelf aAPC therapy product candidate that is engineered to induce a tumor-specific immune response by expanding antigen-specific T cells. RTX-321 expresses hundreds of thousands of copies of an HPV peptide antigen bound to major histocompatibility complex class I proteins, the costimulatory molecule 4-1BBL and the cytokine IL-12 on the cell surface to mimic human T cell-APC interactions.

Fourth Quarter 2020 Financial Results
Net loss for the fourth quarter of 2020 was $40.5 million or $0.50 per common share, compared to $44.5 million or $0.56 per common share in the fourth quarter of 2019.

In the fourth quarter of 2020, Rubius invested $25.6 million in research and development (R&D) related to its novel RED PLATFORM® and towards expanding and advancing its product pipeline, as compared to $30.5 million in the fourth quarter of 2019. This year-over-year decrease was driven primarily by a $6.2 million reduction in costs following the deprioritization of our rare disease pipeline in March 2020. The decrease in rare disease program expenses was offset by $8.4 million of incremental costs to advance our lead cancer programs, including RTX-240 and RTX-321, which were principally related to costs incurred for our Phase 1/2 clinical trial of RTX-240 for the treatment of solid tumors, including clinical CRO and internal manufacturing costs, as well as to costs incurred for IND-enabling activities and clinical startup costs for RTX-321. Additionally, R&D expenses not allocated to programs were reduced by $7.1 million driven primarily by a shift in manufacturing activities towards the technical development and production of clinical supply for our oncology programs and the shift in discovery activities from the development of clinical candidates towards work to enable more advanced programs.

G&A expenses were $14.1 million during the fourth quarter of 2020, as compared to $14.9 million for the fourth quarter of 2019. The lower costs were principally driven by a reduction in stock-based compensation expense.

Full Year 2020 Financial Results
Net loss for the full year 2020 was $167.7 million or $2.08 per common share, compared to $163.5 million or $2.08 per common share for the full year 2019.

For the full year 2020, Rubius invested $116.1 million in R&D related to its novel RED PLATFORM® and towards expanding and advancing its product pipeline, compared to $112.4 million for the full year 2019. The year-over-year increase was driven by $36.6 million of incremental costs to advance our lead cancer programs, including RTX-240 and RTX-321, which were principally related to costs incurred for our Phase 1/2 clinical trial of RTX-240 for the treatment of solid tumors, including clinical CRO and internal manufacturing costs, as well as to costs incurred for preclinical, IND-enabling activities and clinical startup costs for RTX-321. The increase in cancer program costs was offset by a $19.0 million decrease in expenses related to our rare disease pipeline following the deprioritization of these programs in March 2020. Additionally, costs not allocated to programs were reduced by $13.9 million driven primarily by a shift in manufacturing activities towards the technical development and production of clinical supply for our oncology programs and the shift in discovery activities from the development of clinical candidates towards work to enable more advanced programs.

G&A expenses were $50.3 million during the twelve months of 2020, as compared to $57.2 million for the same period in 2019. The lower costs were principally driven by a reduction in stock-based compensation expense.

Cash Position
As of December 31, 2020, cash, cash equivalents and investments were $176.3 million as compared to $283.3 million as of December 31, 2019, providing Rubius with a cash runway into the first quarter of 2022. The Company has the ability to extend runway into the middle of 2022 by scaling back certain activities. During 2020, the Company used $127.8 million of cash to fund operations and $5.5 million to fund capital expenditures, consisting of payments for assets purchased in 2019 related to our manufacturing facility in Rhode Island, as well as computer and laboratory equipment purchased in 2020 for both of our locations. In addition, during 2020 the Company drew down the third and final tranche of $25.0 million pursuant to its $75.0 million loan agreement with Solar Capital.

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 cancer and autoimmune diseases by leveraging two distinct therapeutic modalities — potent

cell-cell interaction and tolerance induction. Rubius Therapeutics was recently named among the Top Places to Work in Massachusetts by the Boston Globe, and its manufacturing site was recently named 2020 Top 5 Best Places to Work in Rhode Island among medium-sized companies by Providence Business News. For more information, visit www.rubiustx.com, follow us on Twitter or LinkedIn or like us on Facebook.

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 our expectations regarding the therapeutic potential of our pipeline of Red Cell Therapeutics, including RTX-240 for the treatment of patients with relapsed/refractory or locally advanced solid tumors and RTX-321 for the treatment of HPV 16-positive cancers, our expectations regarding the timing, enrollment, additional data from and success of the current and future cohorts and phases of the clinical trial of RTX-240, our expectations regarding the biological effects of RTX-240 on innate and adaptive immunity, including activation and increased numbers of NK cells and T cells in the clinical trial of RTX-240, and our expectations regarding the full data set from the Phase 1 clinical trial and its ability to unlock the potential of the RED PLATFORM across our entire pipeline of Red Cell Therapeutics for the treatment of cancer, including our expectations regarding the therapeutic potential of RTX-321, timelines related to the Phase 1 clinical trial of RTX-321, our expectations regarding our ability to expand manufacturing capabilities, our expectations regarding the potential shelf life of our frozen drug substance for RTX-321, our expectations regarding our cash runway 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 September 30, 2020, and subsequent filings with the SEC and risks and uncertainties related to the severity and duration of the impact of COVID-19 on our business and operations. 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.

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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,	
	2020	2019	2020	2019
Revenue	$	$	$	$
Operating expenses:				
Research and development	25,616	30,500	116,107	112,419
General and administrative	14,100	14,928	50,341	57,182
Total operating expenses	39,716	45,428	166,448	169,601
Loss from operations	(39,716)	(45,428)	(166,448)	(169,601)
Other income (expense), net	(823)	956	(1,283)	6,143
Net loss	$ (40,539)	$ (44,472)	$ (167,731)	$ (163,458)
Net loss per share, basic and diluted	$ (0.50)	$ (0.56)	$ (2.08)	$ (2.08)
Weighted average common shares outstanding, basic and diluted:	80,961,343	79,671,342	80,624,608	78,688,878

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

	December 31, 2020	December 31, 2019
Cash, cash equivalents and investments	$ 176,287	$ 283,287
Total assets	277,794	394,841
Total liabilities	136,234	120,628

Total stockholders' equity	141,560	274,213

Source: Rubius Therapeutics