Rubius Therapeutics Reports Second Quarter 2020 Financial Results and Positive Progress Across Pipeline

August 10, 2020

Strong Momentum in Dosing Patients and Manufacturing Clinical Supply for RTX-240 Phase 1/2 Solid Tumor Clinical Trial

Investigational New Drug Application on Track by Year-End for RTX-321 for HPV-Positive Cancers

CAMBRIDGE, Mass., Aug. 10, 2020 (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 2020 financial results and provided an overview of operational progress.

“Rubius Therapeutics is making excellent progress in advancing our pipeline of Red Cell Therapeutics™, including the completion of dosing of the second cohort in the RTX-240 Phase 1/2 solid tumor clinical trial with no observed adverse events to date attributed to RTX-240. As we enter the higher-level dose cohorts, we expect to see the biological effects of RTX-240 on innate and adaptive immunity, including activation and increased numbers of NK cells and T cells. We are continuing to leverage virtual capabilities to engage our clinical trial sites with a concentration on oncology-focused centers, which have been less impacted than hospitals by the ongoing pandemic. Ensuring patient, clinician and employee safety remains our top priority,” said Pablo J. Cagnoni, M.D., chief executive officer of Rubius Therapeutics. “During the second quarter, we presented preclinical data supporting our lead artificial antigen-presenting cell program, RTX-321, at the American Association of Cancer Research and American Society of Gene and Cell Therapy Virtual Annual Meetings. Taken together, these data suggest that RTX-321 may lead to durable responses in patients with HPV 16-positive cancers. We are on track to file an Investigational New Drug application for RTX-321 for the treatment of HPV-positive cancers by year-end.”

Second Quarter Highlights and Upcoming Milestones

• Dosing of the first two cohorts completed in the Phase 1/2 solid tumor clinical trial with no adverse events observed to date that were attributed to RTX-240.

- As Rubius enters higher-level dose cohorts, it expects to see the biological effects of RTX-240 on innate and adaptive immunity, including activation and increased numbers of NK cells and T cells.
- The Company continues to leverage virtual capabilities to engage clinical trial sites and is concentrating on oncology-focused centers to enroll patients during the pandemic, while ensuring that patient and clinician safety is a top priority.
- Rubius’ fully owned manufacturing facility in Smithfield, RI, continues to successfully manufacture clinical supply of RTX-240.

• Rubius highlighted preclinical data for RTX-321 at the American Association of Cancer Research (AACR) Virtual Annual Meeting II and the American Society of Gene and Cell Therapy (ASGCT) 23rd Annual Meeting, supporting its lead artificial antigen-presenting cell program, RTX-321, for the treatment of HPV 16-positive tumors.

- At AACR, the preclinical data suggested that RTX-321 may promote epitope spreading, meaning that RTX-321 may induce the expansion of an immune response to secondary epitopes, or antigens, that are not expressed on RTX-321. This finding is important because it suggests that RTX-321 may create a broad and effective immune response against multiple tumor antigens. Additionally, the preclinical surrogate of RTX-321 induced tumor-specific memory, potentially enabling the body to remember a cancer’s identity, which is critical to providing long-term protection from recurrence of the tumor.
- At ASGCT, preclinical in vitro data demonstrated that RTX-321 can expand the different cell populations - effector and long-lived memory CD8+ cells - that are critical for delivering and maintaining long-term, anti-tumor responses in patients.

Second Quarter Financial Results

Net loss for the second quarter of 2020 was $37.9 million or $0.47 per common share, compared to $39.4 million or $0.50 per common share in the second quarter of 2019.

In the second quarter of 2020, Rubius invested $26.1 million in research and development (R&D) related to its novel RED PLATFORM® and towards expanding and advancing its product pipeline, as compared to $27.5 million in the second quarter of 2019. The year-over-year decrease was driven by a $7.0 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 $8.8 million in incremental spend to advance our cancer programs, including costs associated with our Phase 1/2 clinical trial for RTX-240 for the treatment of solid tumors as well as costs associated with preclinical and IND-enabling activities for RTX-321. In addition, R&D expenses not allocated to programs decreased by $3.2 million driven by a $2.2 million reduction in laboratory supplies, research materials and

contract research as pilot-scale manufacturing and discovery activities shifted to support clinical programs. Other costs not allocated to programs also decreased resulting primarily from a decrease in stock-based compensation expense.

G&A expenses were $11.6 million during the second quarter of 2020, as compared to $13.8 million for the second quarter of 2019. The lower costs were principally driven by a reduction in stock-based compensation expense.

Six Month Financial Results

Net loss for the first six months of 2020 was $86.3 million or $1.07 per common share, compared to $72.0 million or $0.92 per common share in the first six months of 2019.

In the six months ended June 30, 2020, Rubius invested $62.3 million in R&D related to its novel RED PLATFORM® and towards expanding and advancing its product pipeline, as compared to $48.4 million in the first six months of 2019. The year-over-year increase was driven by $18.7 million of incremental costs to advance our cancer programs, including costs incurred for our Phase 1/2 clinical trial for RTX-240 for the treatment of solid tumors as well as costs of preclinical and IND-enabling activities for RTX-321. The increase in cancer program costs was offset by a $5.3 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 increased by $0.5 million, primarily driven by R&D headcount growth.

G&A expenses were $24.3 million during the first six months of 2020, as compared to $27.3 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 June 30, 2020, cash, cash equivalents and investments were $236.5 million as compared to $283.3 million as of December 31, 2019, providing Rubius with a cash runway into 2022. During 2020, the Company used $68.6 million of cash to fund operations and $3.9 million to fund capital expenditures, consisting mostly of payments for assets purchased in 2019. In addition, during the second quarter, 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, Inc. (Nasdaq:RUBY) is 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™. The Company's proprietary RED PLATFORM® genetically engineers and cultures Red Cell Therapeutics to create selective, potent and off-the-shelf allogeneic cellular therapies for the potential treatment of cancer and autoimmune diseases. Rubius' initial product candidates are designed to activate and expand immune system function to fight cancer and modulate the immune system to induce tolerance for the treatment of autoimmune diseases. 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 the, our expectations regarding the therapeutic potential of our Red Cell Therapeutics, including RTX-240 for the treatment of patients with relapsed/refractory or locally advanced solid tumors, our expectations regarding the timing, enrollment, data from and success of the 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 higher-level dose cohorts in the clinical trial of RTX-240, our expectations regarding the therapeutic potential of RTX-321, the timelines for us to file an IND 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 June 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.

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,	
	2020	2019	2020	2019
Revenue	$ -	$ -	$ -	$ -
Operating expenses:				
Research and development	26,096	27,518	62,282	48,389
General and administrative	11,601	13,767	24,265	27,302

Total operating expenses	37,697	41,285	86,547	75,691
Loss from operations	(37,697)	(41,285)	(86,547)	(75,691)
Other income (expense), net	(157)	1,895	207	3,720
Net loss	$ (37,854)	$ (39,390)	$ (86,340)	$ (71,971)
Net loss per share, basic and diluted	$ (0.47)	$ (0.50)	$ (1.07)	$ (0.92)
Weighted average common shares outstanding, basic and diluted:	80,481,756	78,396,714	80,376,830	77,972,757

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

	June 30, 2020	December 31, 2019
Cash, cash equivalents and investments	$ 236,538	$ 283,287
Total assets	344,430	394,841
Total liabilities	138,956	120,628
Total stockholders' equity	205,474	274,213

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