Rubius Therapeutics Reports Second Quarter 2022 Financial Results and Provides Business Update

August 9, 2022

New Preclinical Data Presented at the FOCIS 2022 Annual Meeting Demonstrating Prevention of Type 1 Diabetes in a Stringent Preclinical Model

Initial Clinical Results Expected in 2H'22 from Phase 1 Arm of RTX-240 + Pembrolizumab in Advanced Solid Tumors and Expansion Cohorts in Non-Small Cell Lung Cancer (NSCLC) and Renal Cell Carcinoma (RCC)

Dose Escalation Continues in Phase 1/2 Clinical Trial of RTX-224 in Select Advanced Solid Tumors with No Dose-Limiting Toxicities Observed to Date; Initial Clinical Results Expected by Year-End or During 1Q'23

CAMBRIDGE, Mass., Aug. 09, 2022 (GLOBE NEWSWIRE) -- Rubius Therapeutics, Inc. (Nasdaq: RUBY), a clinical-stage biopharmaceutical company that is biologically engineering red blood cells to create an entirely new class of cellular medicines called Red Cell Therapeutics™ for the treatment of cancer and autoimmune diseases, today reported second quarter 2022 financial results and provided a business update.

"We continue to advance enrollment in the NSCLC and RCC expansion cohorts of our Phase 1 clinical trial of RTX-240 in combination with pembrolizumab, with initial clinical results expected in the second half of 2022, including data in advanced solid tumors and data from the initial patients with NSCLC and RCC enrolled in the study. Enrollment also progressed in the Phase 1/2 clinical trial of RTX-224 in select advanced solid tumors, with initial results expected by year-end or in the first quarter of 2023," said Pablo J. Cagnoni, M.D., president and chief executive officer. "Also, during the quarter, we presented new preclinical data, as part of our type 1 diabetes program, that demonstrated prevention of diabetes and bystander suppression in the stringent non-obese diabetes preclinical model. We plan to select a clinical candidate for our type 1 diabetes program later this year."

Recent Highlights

Oncology

RTX-240 + Pembrolizumab in Advanced Solid Tumors

RTX-240 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 CD8+ memory T cells to generate an anti-tumor response.

- Advanced enrollment in the expansion cohorts of the Phase 1 combination arm of RTX-240 plus pembrolizumab in patients with advanced solid tumors
    - Enrolling up to 20 patients who have experienced disease progression with 1-2 prior treatment regimens in the metastatic setting in preparation for a future Phase 2 clinical trial of RTX-240 in combination with pembrolizumab in an earlier line of therapy
    - Initial clinical results in advanced solid tumors and data from the initial enrolled NSCLC and RCC patients expected in the second half of 2022

RTX-224

RTX-224 is an allogeneic, off-the-shelf cellular therapy product candidate that is engineered to express hundreds of thousands of copies of 4-1BBL and IL-12 on the cell surface. In contrast to RTX-240, RTX-224 is designed as a broad immune agonist of both adaptive and innate responses, activating CD8+ and CD4+ T cells, promoting antigen presentation and activating and expanding NK cells. It is expected to produce a broad and potent anti-tumor T cell response, an innate immune response and have anti-tumor activity in those tumor types with known sensitivity to T cell killing, including tumor types with high mutational burden, PD-L1 expression and prior activity of checkpoint inhibitors.

- Continued dose escalation in the Phase 1/2 clinical trial of RTX-224 in select relapsed/refractory or locally advanced solid tumors, including non-small cell lung cancer, cutaneous melanoma, head and neck squamous cell carcinoma, urothelial (bladder) carcinoma and triple-negative breast cancer
- Initial clinical results are expected by year-end or during the first quarter of 2023

Autoimmunity

Type 1 Diabetes

RCT product candidates are engineered to express specific autoantigens that are the target of immune responses which cause autoimmune disease.

This approach takes advantage of the natural tolerogenic properties of red blood cells. RCTs are designed to suppress, modulate or eliminate disease-causing cells. Type 1 diabetes is a T-cell driven autoimmune disease with well-defined antigens, making it an ideal disease indication for this antigen-specific tolerance approach.

In June, the Company presented new preclinical data for its type 1 diabetes program at the FOCIS 2022 Annual Meeting in San Francisco:

- Demonstrated tolerance induction and bystander suppression in stringent type 1 diabetes preclinical models
- From ongoing experiment, showed new efficacy data in the NOD preclinical model
  - By increasing to 3 doses administered and optimizing the dosing schedule, bystander suppression was achieved at 25 weeks by delivering only two antigens, indicating disease prevention caused by many autoantigens
- Established efficacy in the BDC2.5 adoptive transfer model with data supporting that repeated dosing extends duration of disease protection, reverses established inflammation, which is important for the treatment of existing autoimmunity, and induces two types of regulatory T cells, resulting in protection against re-challenge
- These findings are potentially translatable beyond type 1 diabetes to multiple autoimmune diseases, including multiple sclerosis and celiac disease

Leadership Update

In July 2022, Rubius announced that Susanne Schaffert, Ph.D., joined its board of directors. Dr. Schaffert most recently served as President of Novartis Oncology and brings more than 25 years of experience across clinical development, marketing and sales, finance and commercialization in the global pharmaceutical and biotechnology industries, with a focus on oncology, immuno-oncology and cell therapy.

Anticipated 2022 Catalysts and Operational Objectives

To evaluate the full potential of RTX-240, Rubius' other oncology programs and the RED PLATFORM, Rubius plans to execute several critical milestones in the near term and, based on its current cash and cash equivalents, including borrowings under its credit facility, has sufficient cash runway into the second half of 2023:

- Report initial Phase 1 clinical results for RTX-240 in combination with pembrolizumab in advanced solid tumors and data from the initial enrolled NSCLC and RCC patients in the second half of 2022;
- Select a clinical candidate for the first autoimmune program in type 1 diabetes during the second half of 2022; and
- Report initial Phase 1 clinical results for RTX-224 for the treatment of advanced solid tumors by year-end or during the first quarter of 2023.

Second Quarter Financial Results

Net loss for the second quarter of 2022 was $44.2 million or $0.49 per common share, compared to $50.2 million or $0.56 per common share in the second quarter of 2021.

In the second quarter of 2022, Rubius invested $33.0 million in research and development (R&D) related to its novel RED PLATFORM® and towards expanding and advancing its product pipeline, as compared to $36.1 million in the second quarter of 2021. The year-over-year decrease was principally due to a decrease in direct costs of $2.9 million related to the deprioritization of RTX-321 and RTX-240 AML and monotherapy studies. We expect these costs to continue to decrease in future periods. This decrease was partially offset by an increase in clinical costs related to RTX-224. Platform development, early-stage research and unallocated expenses decreased by $0.1 million primarily due to a decrease of $0.8 million in stock-based compensation expense related to a reduction in the market price of our common stock, resulting in a lower valuation of stock options granted in 2022, and a decrease of $0.3 million in facility-related and other expenses due to lower spend on non-capitalized software costs in the current year. These decreases were partially offset by an increase of $0.4 million in contract research and development related to drug discovery activities and platform development. Additionally, personnel-related costs increased by $0.5 million to support our prioritization of clinical programs.

G&A expenses were $9.9 million during the second quarter of 2022, as compared to $13.9 million for the second quarter of 2021. The lower costs were primarily due to a decrease in stock-based compensation expense of $3.7 million, which was driven by stock option awards that fully vested during the second half of 2021 and first half of 2022, as well as a reduction in the market price of our common stock, resulting in a lower valuation of stock options granted in 2022.

Six Month Financial Results

Net loss for the first six months of 2022 was $96.7 million or $1.07 per common share, compared to $92.5 million or $1.08 per common share in the first six months of 2021.

In the six months ended June 30, 2022, Rubius invested $71.3 million in R&D related to its novel RED PLATFORM® and towards expanding and advancing its product pipeline, as compared to $63.7 million in the first six months of 2021. The year-over-year increase was driven primarily by an increase in direct costs of $2.1 million related to an increase in preclinical and clinical costs associated with RTX-224 and RTX-240, principally due to clinical research organization, or CRO, costs and internal manufacturing costs. This increase was partially offset by a decrease in clinical costs related to RTX-321 due to start-up activities in the prior period and current period deprioritization of clinical development. We expect these costs to decrease in future periods. Platform development, early-stage research and unallocated expenses increased by $5.4 million principally due an increase of $2.9 million in personnel-related costs related to headcount increases through the second half of 2021 to support operations. Additionally, increases of $1.7 million in contract research and development and $1.0 million in laboratory supplies and research materials were related to drug discovery activities and platform development. These increases were partially offset by a decrease of $0.4 million in facility-related and other expenses due to lower spend on non-capitalized software costs and a reduction in building operating costs in the current year.

G&A expenses were $22.5 million during the first six months of 2022, as compared to $27.1 million for the same period in 2021. The lower costs were due to a decrease in stock-based compensation expense of $5.0 million, which was driven by stock option awards that fully vested during the second half of 2021 and first half of 2022, as well as a reduction in the market price of our common stock, resulting in a lower valuation of stock options granted in 2022. The decrease was partially offset by an increase in personnel-related expenses of $0.3 million driven by additions to headcount in our general and administrative function.

Cash Position

As of June 30, 2022, cash, cash equivalents and investments were $140.7 million (including $75 million in borrowings under its credit facility), compared to $225.8 million as of December 31, 2021. During the first six months of 2022, the Company used $81.4 million of cash to fund operations, $78.4 million to purchase investments and $4.2 million to fund capital expenditures, consisting mostly of renovation costs incurred at our manufacturing facility.

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,	
	2022	2021	2022	2021
Revenue	$ -	$ -	$ -	$ -
Operating expenses:				
Research and development	32,998	36,072	71,297	63,749
General and administrative	9,908	13,851	22,471	27,091
Total operating expenses	42,906	49,923	93,768	90,840
Loss from operations	(42,906)	(49,923)	(93,768)	(90,840)
Other income (expense), net	(1,335)	(257)	(2,885)	(1,670)
Net loss	$ (44,241)	$ (50,180)	$ (96,653)	$ (92,510)
Net loss per share, basic and diluted	$ (0.49)	$ (0.56)	$ (1.07)	$ (1.08)
Weighted average common shares outstanding, basic and diluted:	90,257,524	89,517,784	90,203,586	85,936,079

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

	June 30, 2022	December 31, 2021
Cash, cash equivalents and investments	$ 140,713	$ 225,848
Total assets	229,082	318,021
Total liabilities	133,542	139,239
Total stockholders' equity	95,540	178,782

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 biologically 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 named among the 2021 Top Places to Work in Massachusetts by the Boston Globe, and its manufacturing site was recently named 2022 Best Places to Work in Rhode Island 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 beliefs about Rubius' execution across preclinical and clinical development, Rubius' plans and expected timing to present clinical results for RTX-224 and RTX-240, beliefs about the opportunities for and advantages of our drug candidates, plans and timing to execute on critical milestones and our ability to fund those activities, our interpretations of data, including as to the efficacy of our product candidates, expectations regarding the therapeutic potential and safety profile of our pipeline candidates, beliefs about patients' needs, expectations for our cash position and runway, as well as beliefs about our manufacturing plans and accomplishments. 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, risks related to the substantial doubt about our ability to continue as a going concern given that we currently do not have adequate financial resources to fund our forecasted operating costs for at least twelve months and have significant debt outstanding, our ability to pursue and secure financing to fund our operations, those risks and uncertainties related to the development of our Red Cell Therapeutic product candidates and their therapeutic potential, our ability to execute on our plans and expectations, our analyses of clinical and

preclinical data and other risks identified in our filings with the U.S. Securities and Exchange Commission (SEC), including our Annual Report on Form 10-K for the year ended December 31, 2021 and subsequent filings with the SEC, including our Quarterly Report on Form 10-Q for the quarter-ended June 30, 2022, which will be filed on or about the date hereof, 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.

Contacts:
Investors
Lori Murray, Chief Corporate Affairs Officer
lori.murray@rubiustx.com

Media
Marissa Hanify, Director, Corporate Communications
marissa.hanify@rubiustx.com

Source: Rubius Therapeutics