Rubius Therapeutics Announces Strategic Update

September 13, 2022

Company to Focus on Advancing Next Generation Red Blood Cell-Based Cell Conjugation Platform

Initiating Cost-Saving Measures Through Corporate Restructuring; Cash Runway Extended to End of 2023

Discontinuing Ongoing Phase 1 Clinical Trials of RTX-240 and RTX-224 in Advanced Solid Tumors

Investor Teleconference and Webcast Scheduled Today at 7:30 a.m. EDT

CAMBRIDGE, Mass., Sept. 13, 2022 (GLOBE NEWSWIRE) -- Rubius Therapeutics, Inc. (Nasdaq: RUBY), a biopharmaceutical company that is developing an entirely new class of cellular medicines called Red Cell Therapeutics™ for the treatment of cancer and autoimmune diseases, today announced plans to restructure the Company and align resources to advance its next generation red blood cell-based cell conjugation platform.

“With more than 80 patients dosed across three clinical trials to date, Rubius Therapeutics has demonstrated that engineered red blood cells can be manufactured at scale, safely administered and activate a patient’s immune system, resulting in clinical benefit in certain cancer patients, including evidence of tumor shrinkage and prolonged stable disease in PD-(L)-1 refractory solid tumors,” said Pablo J. Cagnoni, M.D., president and chief executive officer of Rubius Therapeutics. “Based on these early findings, we firmly believe in the potential of Red Cell Therapeutics for the treatment of cancer and autoimmunity. Following careful review of recent technical progress in an alternative format for making Red Cell Therapeutics, we believe that this process has the potential for substantive improvements over our existing platform, and, therefore, continued investment in our two current clinical candidates is no longer justified. Instead, we plan to focus on advancing a next generation red blood cell platform that utilizes cell conjugation to potentially improve upon the existing benefits of the RED PLATFORM, with the potential for greater efficacy and enhanced versatility, while maintaining our favorable tolerability profile. As a result, we have decided to discontinue our ongoing clinical trials of RTX-240 and RTX-224 for the treatment of advanced solid tumors and restructure the organization to support advancing drug candidates based on the next generation platform. I would like to express my sincere gratitude to the patients who participated in our clinical trials, the investigators who partnered with us in this endeavor and our employees for their immense dedication and contributions that have brought us to where we are today.”

Next Generation Red Blood Cell-Based Conjugation Platform Overview

The new platform is expected to improve upon the existing benefits of the RED PLATFORM, with the potential for greater efficacy and enhanced versatility, while maintaining a favorable tolerability profile, and reduce the complexity and cost of generating cells by leveraging chemical conjugation to produce Red Cell Therapeutics. Cell conjugation creates a covalent link between the cell surface and the molecule of interest. This approach is intended to:

- deliver a higher effective dose by enabling a longer circulation time and/or administering a higher cell dose;
- be more versatile, enabling the conjugation of different payloads, immunomodulatory agents, small molecules and proteins on the cell for enhanced potency; and
- reduce the cost of goods manufacturing by utilizing blood-banked RBCs versus biologically engineering and differentiating early progenitor cells into reticulocytes that express proteins

These attributes are expected to result in greater efficacy, a similar safety profile given the restricted biodistribution of RBCs to the spleen and vasculature and a significant reduction in overall cost structure.

Business & Strategy Update

To enable continued investment in the new platform, the Company is restructuring its business and implementing a series of cost-saving measures, which extends the Company’s cash runway until the end of 2023. These measures include:

- Implementing a 75% reduction in force, primarily focused on clinical development, manufacturing and general and administrative;
- Discontinuing its ongoing Phase 1 clinical trials of RTX-240 and RTX-224 for the treatment of advanced solid tumors; and
- Exploring the sale of its manufacturing facility in Smithfield, Rhode Island
    - Patients still on trial will continue to be dosed until disease progression or discontinuation (n=6)

The Company will maintain its robust technical development and preclinical oncology and autoimmunity research capabilities to advance the new platform and related preclinical programs.

Overview of Current Clinical Programs

Phase 1 Clinical Trial of Monotherapy RTX-240

As of September 12, 2022, 36 patients have been dosed in the monotherapy arm of the Phase 1/2 clinical trial of RTX-240 in advanced solid tumors. One patient with renal cell carcinoma remains on study with stable disease for more than one year after developing progressive disease on prior treatment with nivolumab. This ongoing patient was dose-escalated from the 3e10 x 3 + 1e10 Q3W dose level (n=6) to the 5e10 Q3W dose level after Cycle 12. Seven patients were dosed at 5e10 Q3W with one anal cancer patient experiencing stable disease for 5 months before disease progression. RTX-240 continued to be well tolerated with no treatment-related Grade 3/4 adverse events (AEs) and no dose-limiting toxicities.

Phase 1 Clinical Trial of RTX-240 + Pembrolizumab in Advanced Solid Tumors

Fourteen patients were dosed in the “all comers” dose-escalation portion of the Phase 1 clinical trial evaluating RTX-240 in combination with pembrolizumab in advanced solid tumors. Four patients had stable disease of greater than 12 weeks with one colorectal cancer patient remaining on study with stable disease of greater than 4 months. Two additional patients have been dosed in the non-clear cell RCC (nccRCC) and non-small cell lung cancer (NSCLC) expansion cohorts. Both patients remain on treatment with one nccRCC patient with stable disease greater than 12 weeks and one NSCLC patient who is not yet evaluable for response. The combination of RTX-240 with pembrolizumab continued to be well tolerated with no treatment or investigator-identified immune-related Grade 3/4 AEs and no dose-limiting toxicities.

Phase 1 Clinical Trial of RTX-224 in Select Advanced Solid Tumors

Seven patients have been dosed across two dose cohorts in the Phase 1 clinical trial evaluating RTX-224 in select 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. One patient dosed at the 5e8 Q3W dose level with melanoma remains on study and is not yet evaluable for response. There have been no treatment-related Grade 3/4 AEs and no dose-limiting toxicities.

Investor Teleconference & Webcast

An audio webcast will be available on the Events and Presentations page within the Investors and Media section of the Rubius Therapeutics website and can be directly accessed here. An archived webcast will be accessible for 90 days after the event.

About Rubius Therapeutics

Rubius Therapeutics is a biopharmaceutical company that is developing an entirely new class of cellular medicines called Red Cell Therapeutics™ for the treatment of cancer and autoimmune diseases. The Company 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 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 and expectation for the restructuring plan described in this release, including associated costs, cost savings and timing, as well as our beliefs about the next generation red blood cell platform, including that it will potentially improve upon the existing benefits of the RED PLATFORM, and its potential for greater efficacy and enhanced versatility. 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 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 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 12 months; our ability to execute on and realize the expected benefits of the restructuring plan described in this release; the amount of debt we have outstanding and the lender’s rights under our loan facility, including the lender’s ability to accelerate amounts outstanding under the loan facility, or exert control over our cash accounts in connection with certain events of default, including a material adverse change in our business; our ability to pursue and secure financing to fund our operations; our ability to maintain our listing on the Nasdaq Stock Market, particularly in light of our recently disclosed deficiencies; those risks and uncertainties related to the development of the our Red Cell Therapeutic product candidates and their therapeutic potential; risks related to our ability to execute on our plans and expectations and 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 Quarterly Report on Form 10-Q for the quarter-ended June 30, 2022 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 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.

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Source: Rubius Therapeutics