Rubius Therapeutics Announces 2020 Objectives and Provides Operational Update at the 38th Annual J.P. Morgan Healthcare Conference

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RTX-240 Investigational New Drug Application for Cancer on File with U.S. Food and Drug Administration

Rubius Manufacturing Facility Operational Nine Months Ahead of Schedule and
Ready to Supply Future Clinical Trials

Focus on Clinical Development to Advance Three Distinct Therapeutic Approaches Across
Rare Enzyme Deficiencies and Cancer

CAMBRIDGE, Mass., Jan. 13, 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 announced 2020 objectives and operational updates. Pablo J. Cagnoni, M.D., chief executive officer, will present these updates and review 2019 accomplishments today at 9:00 a.m. PST / 12:00 p.m. EST at the 38th Annual J.P. Morgan Healthcare Conference in San Francisco, California.

“Rubius Therapeutics made significant strides in 2019 to strengthen the organization and advance our pipeline of Red Cell Therapeutics™. We filed our first-ever Investigational New Drug (IND) applications for RTX-134 for the treatment of phenylketonuria and RTX-240 for the treatment of solid tumors, operationalized our fully-owned manufacturing facility nine months ahead of schedule and strengthened our leadership team,” said Pablo J. Cagnoni, M.D., chief executive officer. “As we look ahead in 2020, we are focused on prosecuting our lead programs that represent three distinct therapeutic approaches – our enzyme replacement therapy with RTX-134, broad stimulation of the immune system with RTX-240 and antigen-specific immune stimulation with RTX-321 for the treatment of HPV-positive cancers. We plan to announce when we dose the first patient enrolled in the RTX-134 clinical trial and expect to provide an update on timing for reporting initial clinical results at that time. We also plan to announce when we begin dosing patients in the RTX-240 Phase 1 clinical trial, and, by year-end, we expect to file an IND for RTX-321.”

2020 Objectives and Operational Updates

RTX-134 for the Treatment of Phenylketonuria (PKU)

RTX-134 is an allogeneic cellular therapy product candidate that is genetically engineered to express the enzyme phenylalanine ammonia-lyase (PAL) inside RTX-134 to degrade toxic levels of phenylalanine that accumulate due to a deficiency in the phenylalanine hydroxylase enzyme.

- Rubius plans to announce when the first patient has been dosed in the Phase 1b clinical trial evaluating RTX-134 in adults with PKU and expects to provide an update on timing for reporting of initial clinical results at that time.
- The initial clinical results expected are preliminary safety, longevity of RTX-134 in circulation and proof-of-mechanism as measured by the production of trans-cinnamic acid, a metabolite of phenylalanine when degraded by PAL.

RTX-240 for the Treatment of Solid Tumors

RTX-240 is an allogeneic cellular therapy product candidate engineered to replicate the human immune system by broadly stimulating the adaptive and innate immune systems to generate an immune response. RTX-240 expresses 4-1BBL and IL-15TP, a fusion of IL-15 and IL-15 receptor alpha, on the cell surface with the goal of improving anti-tumor activity and overcoming resistance to immunotherapy in patients with solid tumors.

- Rubius has filed an IND with U.S. FDA for RTX-240 for the treatment of cancer and plans to announce when the first patient has been dosed.
- GMP grade RTX-240 is expected to be manufactured at Rubius’ fully-owned manufacturing facility.

RTX-321 for the Treatment of HPV-Positive Tumors

RTX-321 is an allogeneic artificial antigen-presenting cellular therapy product candidate that is engineered to induce a tumor-specific immune response by expanding antigen-specific T cells. RTX-321 expresses an HPV peptide antigen bound to MHC I, 4-1BBL and IL-12 on the cell surface to mimic human T cell-APC interactions.

- Rubius plans to file an IND for RTX-321 by year-end.
- GMP grade RTX-321 is expected to be manufactured at Rubius’ fully-owned manufacturing facility.

Additional Manufacturing Update

- Rubius has the potential to significantly expand its manufacturing capabilities at its Smithfield, RI site and plans to stage

additional investments based on future supply needs.

2019 Achievements

- Filed and received clearance of IND for first-ever Red Cell Therapeutic – RTX-134 for the treatment of PKU
- Accelerated Rubius manufacturing facility buildout and GMP operations by nine months to supply future clinical trials
- Filed the first oncology IND for RTX-240 for the treatment of solid tumors and completed engineering runs at Rubius manufacturing facility
- Manufactured clinical-grade RTX-134 at its contract manufacturing organization to support RTX-134 Phase 1b clinical trial
- Presented preclinical oncology data at the 2019 American Association for Cancer Research (AACR) Annual Meeting, AACR-NCI-EORTC International Conference on Molecular Targets and Cancer and the Society for Immunotherapy of Cancer (SITC) 34th Annual Meeting
- Strengthened the leadership team with key strategic hires: chief scientific officer, chief medical officer, chief legal officer, head of business development and strategy and chief quality officer.
- Grew the patent estate to 10 issued U.S. patents, 32 patent families and more than 165 patent applications worldwide.
    - All product candidates (RTX-134, RTX-240, RTX-224 and RTX-321) are covered by three varieties of issued U.S. patents: composition of matter, method of treatment and method of making.

Listen to the Webcast

A live audio webcast of Dr. Cagnoni's presentation will be available today at 9:00 a.m. PST / 12:00 p.m. EST within the Investors & Media section of the Rubius Therapeutics website. An archived replay will be accessible for 90 days following the event.

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, 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 planned 2020 objectives such as the planned timing, recruitment, enrollment and results for our preclinical and clinical activities, including the Phase 1b clinical trial for RTX-134 for the treatment of PKU, our expectations regarding the results from our Phase 1b clinical trial for RTX-134, our ability to further develop our RCT product candidates, our planned timing for additional IND applications our manufacturing process and capabilities, our expectations regarding the therapeutic potential of our RCTs, our ability to meet the objectives of our planned clinical trials and demonstrate the safety and efficacy of our product candidates, our expectations regarding new and continuing members of our leadership team 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 September 30, 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.

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