Rubius Therapeutics Highlights Upcoming 2019 Milestones at the 37th Annual J.P. Morgan Healthcare Conference

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CAMBRIDGE, Mass., Jan. 07, 2019 (GLOBE NEWSWIRE) -- Rubius Therapeutics, Inc. (Nasdaq:RUBY), a biotechnology company that is generating red blood cells and bioengineering them into an entirely new class of cellular medicines, today announced anticipated 2019 development milestones. Pablo J. Cagnoni, M.D., chief executive officer, will present these updates and review 2018 accomplishments today at 9:00 a.m. PT at the 37th Annual J.P. Morgan Healthcare Conference.

“At Rubius Therapeutics, we made tremendous progress in 2018, transforming from a privately-held company into a publicly-traded, fully integrated research and development organization that is advancing a robust pipeline of Red Cell Therapeutics™ for the potential treatment of rare enzyme deficiencies, cancer and autoimmune diseases,” said Dr. Cagnoni. “As we look ahead, 2019 promises to be equally productive for Rubius as we prepare to file our first Investigational New Drug (IND) application for RTX-134 for the treatment of phenylketonuria during the first quarter of 2019, with initial clinical data expected during the second half of the year. Additionally, we plan to file an IND by early 2020 for our lead cancer program, RTX-240 (formerly RTX-212), for the treatment of solid tumors. In total, we expect to file four to five INDs in 2019 and 2020.”

Anticipated 2019 Milestones and Operational Updates

Rare Enzyme Deficiency Disorders Overview
Red Cell Therapeutics (RCTs) are engineered to express therapeutic enzymes within the cell and to shield those enzymes from the immune system. In rare enzyme deficiencies, RCTs are designed to circulate in the blood stream and degrade toxic metabolites that accumulate as a result of these deficiencies. Compared to current therapeutic approaches, RCT product candidates may have a more sustained treatment effect given the 120-day circulating time of red blood cells and may have a lower incidence of immune-driven adverse events, including the formation of neutralizing antibodies to the therapeutic enzyme, which often results in a reduction in efficacy. We expect RCTs to provide a more efficacious treatment option to patients.

RTX-134 for the Treatment of Phenylketonuria (PKU)

- RTX-134 is genetically engineered to express the enzyme phenylalanine ammonia lyase (PAL) inside the RCT to convert phenylalanine into two metabolites, trans-cinnamic acid and ammonia. RTX-134 is designed to treat PKU, a rare disease characterized by a buildup of the neurotoxic amino acid phenylalanine
- IND filing on track for 1Q’19
- Initial data expected in 2H’19 from Part 1 of the RTX-134 Phase 1b/2a clinical trial in adults with PKU
- High-level objectives for the RTX-134 clinical trial:

  ° Part 1: Single-dose administration
  - Preliminary safety
  - Longevity of cells in circulation
  - Production of trans-cinnamic acid as a biomarker of RTX-134’s mechanism of action
  - Dose and schedule selection for Part 2 of the study

  ° Part 2: Multi-dose administration
  - Safety Efficacy – Magnitude of reduction in phenylalanine
  - Dose and schedule optimization

Cancer Overview
RCT product candidates can be engineered to express combinations of co-stimulatory molecules on the cell surface to directly engage T and NK cells to activate the adaptive and innate immune systems. The goal is to stimulate these immune cells to proliferate, activate, migrate and, ultimately, to attack and kill tumors. Rubius Therapeutics’ lead oncology candidates each are designed to stimulate the immune system in different ways and provide several benefits over existing immuno-oncology approaches: 1) the expression of natural ligands in the appropriate conformation and in synergistic combinations is expected to provide a more potent activation of the immune system; 2) RCTs are confined to the vasculature versus administered systemically, which is expected to result in an improved safety profile; and 3) as a result, RCTs are expected to have a broader therapeutic window.

RTX-240 (formerly RTX-212) for the Treatment of Solid Tumors

- RTX-240 co-expresses 4-1BBL and IL-15TP, a fusion of IL-15 and IL-15 receptor alpha, with the goal of improving anti-tumor activity and overcoming resistance to immunotherapy in patients with solid tumors
- IND filing expected by early 2020
- Preclinical data is expected to be presented or published during 2019

RTX-224 for the Treatment of Solid Tumors

- RTX-224 co-expresses 4-1BBL and IL-12 on the cell surface with the goal of improving overall anti-tumor immune response
- In preclinical models, RTX-224 drives deep tumor regressions and improved safety
- Preclinical data is expected to be presented or published during 2019

Autoimmune Diseases Overview
RCT product candidates are engineered to express disease-causing antigens on their surface. When these RCTs are processed by the reticuloendothelial system, the antigens are expected to be re-presented to the immune system. This approach is intended to retrain the immune system, or induce tolerance, to no longer respond to self-antigens as foreign. Restoration of immune tolerance could lead to cures in certain autoimmune diseases.

- First autoimmune disease clinical candidate designed to induce immune tolerance is expected to be selected in 2019

Successful Execution on Key Priorities in 2018

- Purchased 135,000 sq. ft. manufacturing facility in Smithfield, RI and initiated renovations
- Scaled manufacturing of RCTs from 50L to 200L bioreactors
- Transferred RTX-134 manufacturing process to contract manufacturing organization
- Executed three successful financings, including a $101.0 million crossover round, a $254.3 million initial public offering after expenses, and a recent debt financing of up to $75.0 million, which further extends cash runway into 2021
- Generated additional preclinical data with lead oncology candidates, RTX-240 and RTX-224, that suggests a broad therapeutic window for the treatment of cancer
- Increased RCT storage stability from 28 days to 42 days with additional studies ongoing
- Continued to strengthen the board of directors, build a leading scientific team and attract experienced leadership to deliver against Rubius' objectives
- Strengthened internal capabilities in discovery, platform and therapeutic development and manufacturing

About Rubius Therapeutics
Rubius Therapeutics is a biopharmaceutical company that is generating red blood cells and bioengineering them into an entirely new class of cellular medicines. The Company's proprietary RED PLATFORM ® is designed to genetically engineer and culture Red Cell Therapeutics™ that are selective, potent, ready-to-use allogeneic cellular therapies. Rubius is leveraging three distinct therapeutic modalities — cellular shielding, potent cell-cell interaction and tolerance induction for the potential treatment of rare enzyme disorders, cancer and autoimmune diseases. The Company expects to file an Investigational New Drug application during the first quarter of 2019, for its lead program RTX-134 for the treatment of phenylketonuria. For more information, visit **www.rubiustx.com** or follow us on **Twitter** and **LinkedIn**.

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 therapeutic potential of our Red Cell Therapeutics 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 related to the planned timing for our first IND application for RTX-134 for the treatment of phenylketonuria, the planned timing for additional IND applications, the expected timing and progress of preclinical and clinical data for our product candidates, our ability to meet the objectives of our planned clinical trials and demonstrate the safety and efficacy of our product candidates, the planned timing for our selection of an autoimmune disease clinical candidate, our ability to fund the development of our Red Cell Therapeutic product candidates, the development of our Red Cell Therapeutic product candidates and their therapeutic potential, whether and when, if at all, our Red Cell Therapeutic product candidates will receive approval form the U.S. Food and Drug Administration and for which, if any, indications, competition from other biotechnology companies, and other risks identified in our SEC filings, including our Form 10-Q for the quarter ended September 30, 2018, 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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