Rubius Therapeutics Announces First Patient Dosed in Phase 1b Trial of RTX-134 for the Treatment of Patients with Phenylketonuria

January 30, 2020

CAMBRIDGE, Mass., Jan. 30, 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 that the first patient has been dosed in its Phase 1b trial of RTX-134, an allogeneic, off-the-shelf cellular therapy for the potential treatment of patients with phenylketonuria (PKU). The trial is designed to evaluate the safety and pharmacokinetics of RTX-134 in adult patients with PKU.

“Phenylketonuria is a devastating metabolic disorder that can lead to serious cognitive impairment if not properly treated,” said Pablo J. Cagnoni, M.D., chief executive officer of Rubius. “Our ultimate goal in developing RTX-134, the first-of-its-kind, off-the-shelf Red Cell Therapeutic™, is to provide patients with a well-tolerated and effective treatment option that lowers phenylalanine via infrequent administration and allows people with PKU to enjoy a normal diet. The initiation of this trial is a very exciting moment for both Rubius and the patients and caregivers we hope to serve.”

Rubius expects to report initial clinical data from the first patients enrolled in the trial, including preliminary safety, longevity of RTX-134 in circulation and proof-of-mechanism as measured by production of trans-cinnamic acid (TCA), a metabolite of phenylalanine when degraded by phenylalanine ammonia lyase (PAL). The Company plans to provide an update on timing of the initial data as the trial progresses.

About the RTX-134 Phase 1b Clinical Trial

The Phase 1b clinical trial is an open-label, single-dose safety, tolerability and pharmacokinetics study of RTX-134 in up to 12 PKU patients whose phenylalanine (Phe) levels are above 600 µmol/L. The primary objectives of the study are to evaluate the safety and tolerability of RTX-134, correlate dose with percent reduction in serum Phe levels relative to baseline and determine a preliminary dose to achieve serum Phe levels less than 360 µmol/L and 600 µmol/L, respectively. The study will also evaluate the pharmacodynamics of RTX-134, including measurement of TCA and cell circulation time. The Phase 1b clinical trial includes four dose cohorts with two patients treated per dose cohort. The design of the trial provides flexibility to enroll additional patients to planned cohorts or add additional cohorts.

About RTX-134

RTX-134 is a Red Cell Therapeutic product candidate that is genetically engineered to express the enzyme PAL inside the cell. RTX-134 is designed to circulate in the bloodstream and degrade toxic levels of phenylalanine that accumulate due to a deficiency in the phenylalanine hydroxylase (PAH) enzyme. Compared to current therapeutic interventions, RTX-134 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 since PAL is inside RTX-134 and shielded from the immune system. As a result, RTX-134 may provide a well-tolerated, efficacious and convenient treatment option for patients.

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 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 ability to further develop our RCT product candidates, our manufacturing process, our expectations regarding the therapeutic potential of our RCTs, 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.

Contacts:
Lori Melançon
Vice President, Corporate Communications and Investor Relations
+1 (617) 949-5296
lori.melancon@rubiustx.com

Media Contact:
Dan Budwick
1AB
+1 (973) 271-6085
dan@1abmedia.com

Source: Rubius Therapeutics