Rubius Therapeutics Announces Dosing of First Patient with Relapsed/Refractory Acute Myeloid Leukemia in the Ongoing Phase 1/2 Clinical Trial of RTX-240

November 5, 2020

CAMBRIDGE, Mass., Nov. 05, 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 called Red Cell Therapeutics™, today announced that the first patient with acute myeloid leukemia (AML) has been dosed in its ongoing Phase 1/2 clinical trial of RTX-240, an allogeneic cellular therapy product candidate that is being evaluated for the treatment of patients with relapsed/refractory or locally advanced solid tumors or relapsed/refractory AML. The ongoing clinical study of RTX-240 has two Phase 1 arms, one in all solid tumors and the other in relapsed/refractory AML. RTX-240 is engineered to stimulate innate and adaptive immunity by activating natural killer (NK) cells and T cells inside the patient's body to generate a potent anti-tumor immune response.

"A number of studies show that NK cells can exhibit potent anti-tumor activity against AML, but tumor-associated mechanisms often suppress the proper function of NK cells leading to disease progression," said Christina Coughlin, M.D., Ph.D., chief medical officer of Rubius Therapeutics. "When NK cells are restored to their full anti-tumor potential, their cytolytic activity predicts a better long-term outcome for patients with AML. RTX-240 is designed to replicate immune system function by activating and expanding a patient's own NK and T cells in the body to generate an immune response against cancer."

RTX-240 is engineered to simultaneously express hundreds of thousands of copies of the costimulatory molecule 4-1BB ligand (4-1BBL) and the cytokine interleukin-15 (IL-15TP) to broadly stimulate both powerful arms of the immune system to generate anti-tumor immunity.

"Despite advances in the management of AML, the overall outcome for patients with relapsed or refractory disease remains quite poor due to several factors, including lack of disease response to salvage therapy and the overall toxicity of the therapies," said Alexander Spira, M.D., Ph.D., director of the Virginia Cancer Specialists Research Institute and the Phase I Trial Program. "RTX-240 potentially offers a novel way of treating relapsed/refractory AML by using the body's own immune system to attack cancer. We look forward to working with Rubius Therapeutics on this differentiated approach to treating AML."

About Acute Myeloid Leukemia (AML)
AML is a rare and aggressive cancer of the blood and bone marrow, and is the most common form of acute leukemia in adults. AML begins in the bone marrow and most often moves quickly into the blood or other areas of the body.

Refractory AML occurs when patients have received treatment, typically two rounds of chemotherapy, but the disease does not go into remission. Some patients experience remission and then a return of the leukemia, which is referred to as relapsed AML. The overall outcome for patients with relapsed/refractory AML remains poor, with 5-year overall survival of approximately 10 percent. Limited therapeutic options currently exist for these patients.

About the RTX-240 Clinical Trial
Rubius Therapeutics is enrolling patients in a Phase 1/2 open label, multicenter, multidose, first-in-human dose-escalation and expansion study of RTX-240. The study contains two Phase 1 dose escalation arms: one in patients with relapsed/refractory or locally advanced solid tumors and another in patients with relapsed/refractory acute myeloid leukemia. These two Phase 1 arms will determine the safety and tolerability, pharmacokinetics, maximum tolerated dose and a recommended Phase 2 dose and dosing regimen of RTX-240 in patients with solid tumors as well as in patients with relapsed/refractory AML. The trial will also assess the pharmacodynamics of RTX-240 measured by changes in T and NK cell number and function relative to baseline and anti-tumor activity in both patient populations. The study will include an expansion phase in specified tumor types during the Phase 2 portion of the solid tumor arm. The extent to which the COVID-19 pandemic may impact Rubius' ability to enroll patients in the trial will depend on future developments.

About RTX-240
RTX-240 is an allogeneic cellular therapy product candidate that is being evaluated for the treatment of patients with relapsed/refractory or locally advanced solid tumors or relapsed/refractory acute myeloid leukemia. RTX-240 is engineered to simultaneously present hundreds of thousands of copies of the costimulatory molecule 4-1BBL and IL-15TP (trans-presentation of IL-15 on IL-15Rα) in their native forms and is designed to stimulate innate and adaptive immunity by activating NK cells and T cells inside the patient's body to generate an anti-tumor immune response.

About Rubius Therapeutics
Rubius Therapeutics, Inc. (Nasdaq:RUBY) is a clinical-stage biopharmaceutical company that is genetically engineering red blood cells to create an entirely new class of cellular medicines called Red Cell Therapeutics™. The Company's proprietary RED PLATFORM® genetically engineers and cultures Red Cell Therapeutics to create selective, potent and off-the-shelf allogeneic cellular therapies for the potential treatment of cancer and autoimmune diseases. Rubius' initial product candidates are designed to activate and expand immune system function to fight cancer and modulate the immune system to induce tolerance for the treatment of autoimmune diseases. Rubius Therapeutics' manufacturing site was recently named 2020 Top 5 Best Places to Work in Rhode Island among medium-sized companies 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 our expectations regarding the therapeutic potential of our Red Cell Therapeutics, including RTX-240 for the treatment of solid tumors and acute myeloid leukemia, 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, expectations regarding the therapeutic potential of RTX-240 and our clinical study of RTX-240, those risks and uncertainties related to the development of our Red Cell Therapeutic 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 June 30, 2020, 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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Source: Rubius Therapeutics