Rubius Therapeutics Announces First Patient Dosed with RTX-240 in Combination with KEYTRUDA® (pembrolizumab) in Ongoing Phase 1/2 Clinical Trial for the Treatment of Advanced Solid Tumors

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CAMBRIDGE, Mass., June 23, 2021 (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 has been dosed in a new Phase 1 arm of the ongoing Phase 1/2 clinical trial of RTX-240 in combination with KEYTRUDA® (pembrolizumab)[1] for the treatment of patients with relapsed/refractory or locally advanced solid tumors. To be eligible for the trial, patients must have disease that is relapsed or refractory to an anti-PD-1 or PD-L1 therapy.

“RTX-240 is designed to activate and expand a patient’s own immune cells to mount a broad and potent anti-tumor response. Combining an immune agonist with a PD-1 checkpoint inhibitor has the potential to prevent the cancer from evading the immune response. The RTX-240 and pembrolizumab combination is anticipated to drive activated T cells and NK cells into the tumor microenvironment with the potential to overcome resistance to PD-1 inhibition,” said Christina Coughlin, M.D., Ph.D., chief medical officer at Rubius Therapeutics. “Given the favorable emerging safety profile and promising initial clinical activity reported as part of our initial clinical results from the ongoing Phase 1 monotherapy trial of RTX-240 in advanced solid tumors, we believe that the combination with pembrolizumab has the potential to provide significant benefit to patients with disease that is relapsed or refractory to prior anti-PD-1 or PD-L1 therapy.”

“Despite recent advances in cancer immunotherapy, there remains a need for new combination approaches that broaden the benefits of immunotherapy in patients with solid tumors following treatment with checkpoint inhibition,” said Omid Hamid, M.D., Chief of Translational Research and Immunotherapy, Director of the Phase 1 Immuno-Oncology Program of The Angeles Clinic and Research Institute, a Cedars-Sinai Affiliate, and RTX-240 investigator. “The exciting initial safety and efficacy data from the ongoing monotherapy Phase 1 clinical trial make RTX-240 a potentially promising candidate for the treatment of cancer, and we are excited to work with Rubius to evaluate RTX-240 in combination with pembrolizumab.”

RTX-240 is engineered to express a co-stimulatory molecule, 4-1BB ligand, and a cytokine, IL-15TP, on the cell’s surface, and is designed to broadly stimulate the immune system by activating and expanding both natural killer (NK) cells and T cells to generate a potent anti-tumor response. Pembrolizumab is a humanized monoclonal immunoglobulin G4 antibody (IgG4 mAb) with high specificity of binding to the programmed cell death protein-1 (PD-1) receptor, thus inhibiting its interaction with programmed cell death ligand 1 (PD-L1) and programmed cell death ligand 2 (PD-L2). Based on in vitro data, pembrolizumab has high affinity and potent receptor blocking activity for PD-1. Pembrolizumab is indicated for the treatment of patients across several indications as monotherapy and in combination with numerous therapies.

About the RTX-240 Phase 1/2 Clinical Trial
This is a Phase 1/2 open label, multicenter, multidose, first-in-human dose-escalation and expansion study designed to determine the safety and tolerability, pharmacokinetics, maximum tolerated dose and a recommended Phase 2 dose and dosing regimen of RTX-240. 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. The trial has three separate Phase 1 arms: an ongoing monotherapy dose escalation arm in adults with relapsed/refractory or locally advanced solid tumors, an ongoing monotherapy dose escalation arm in adults with relapsed/refractory acute myeloid leukemia, and a combination therapy dose escalation arm with pembrolizumab in adults with relapsed/refractory or locally advanced solid tumors. The monotherapy arm of the trial in advanced solid tumors includes a Phase 2 expansion in specified tumor types.

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 cancer and autoimmune diseases by leveraging two distinct therapeutic modalities — potent cell-cell interaction and tolerance induction. Rubius Therapeutics was named among the 2020 Top Places to Work in Massachusetts by the Boston Globe, and its manufacturing site was recently named 2021 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 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 for the therapeutic potential of our pipeline of Red Cell Therapeutics, including RTX-240 and its potential to expand a patient’s immune cells and related response; expectations for combining an immune agonist with a PD-1 checkpoint inhibitor; anticipated benefits of RTX-240 to overcome resistance to PD-1 inhibition; beliefs that RTX-240 has the potential to provide significant benefit to patients; expectations regarding combination approaches regarding, and the benefits of, immunotherapy; plans and partnerships to evaluate RTX-240 in combination with pembrolizumab; and beliefs about the safety and efficacy profile of RTX-240 and its potential role in the treatment of cancer. 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 Red Cell Therapeutic product candidates, including RTX-240, and their therapeutic potential 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 March 31, 2021, 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 our views as of any subsequent date. We explicitly disclaim any obligation to update any forward-looking statements.

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[1]KEYTRUDA® is a registered trademark of Merck Sharp & Dohme Corp., a subsidiary of Merck & Co., Inc.

Source: Rubius Therapeutics