Rubius Therapeutics Announces First Patient Dosed in Phase 1 Clinical Trial of RTX-321 for the Treatment of HPV 16-Positive Cancers

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CAMBRIDGE, Mass., April 12, 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 the Phase 1 clinical trial of RTX-321 for the treatment of human papilloma virus (HPV) 16-postive cancers. RTX-321 is an allogeneic, off-the-shelf Red Cell Therapeutic product candidate that is engineered as an artificial antigen-presenting cell (aAPC) with a dual mechanism of action: boosting HPV 16-specific CD8+ T cell responses and promoting broad stimulation of both innate and adaptive immune responses. The Phase 1 clinical trial of RTX-321 is enrolling patients with persistent, recurrent, or metastatic, unresectable, HPV 16-positive cancers, including cervical cancer, head and neck squamous cell carcinoma (HNSCC) and anal cancer.

“In preclinical studies, the surrogate model of RTX-321 induced a broad immune response and epitope spreading, suggesting that in patients, an effective immune response could be generated against multiple HPV antigens,” said Christina Coughlin, M.D., Ph.D., chief medical officer of Rubius Therapeutics. “In patients, RTX-321 may also induce tumor-specific memory, potentially enabling the patient’s own immune system to remember a cancer’s identity, which could lead to long-term protection from tumor recurrence. Based on these findings, we believe that RTX-321 may lead to durable responses in patients with HPV 16-positive cancers.”

RTX-321 expresses hundreds of thousands of copies of the costimulatory molecule 4-1BBL, the cytokine IL-12 and an HPV peptide antigen bound to major histocompatibility complex (MHC) class I proteins on the cell surface to mimic human T cell-APC interactions. As part of the manufacturing process, Rubius is producing frozen drug substance for the first time, enabling a truly off-the-shelf cellular therapy product candidate with a potential shelf life of up to several years.

“HPV 16 is the most common high-risk strain of HPV and is known to be associated with various types of cancer, including cervical cancer, head and neck squamous cell carcinoma and anal cancer. For patients with advanced HPV 16-positive cancers, the prognosis remains poor with few treatment options beyond the first-line setting,” said Howard A. “Skip” Burris, III, M.D., president, clinical operations and chief medical officer, Sarah Cannon Research Institute. “RTX-321 offers a new potential option to treat these patients by utilizing the body’s own immune system. We look forward to working with Rubius Therapeutics to develop RTX-321 for the treatment of patients with HPV 16-positive cancers.”

About HPV 16-Positive Cancers
Human papillomavirus (HPV) 16 is associated with approximately 70 percent of cervical cancers, approximately 40 percent of head and neck squamous cell carcinoma (HNSCC) arising in the oropharynx, approximately 25-40 percent of HNSCC arising in other locations and approximately 80-85 percent of anal cancers. A critical need remains for better treatment options for advanced HPV 16-associated cancers. The prognosis remains poor for patients with metastatic disease with few treatment options beyond the first-line setting.

About the RTX-321 Clinical Trial
Rubius Therapeutics is enrolling patients in a Phase 1 open-label, multicenter, monotherapy dose escalation, first-in-human study of RTX-321 for the treatment of patients that are HLA-A*02:01-positive with persistent, recurrent, or metastatic, unresectable, HPV 16-positive cancers, including unresectable cervical cancer (squamous, adeno, or adenosquamous histology), head and neck squamous cell carcinoma (including of the nasal and oral cavities, larynx, hypopharynx, nasopharynx, and oropharynx) and squamous cell cancer of the anal canal that is not amenable to curative therapy. The purpose of the trial is to determine the safety and tolerability, recommended phase 2 dose and pharmacology, and antitumor activity of RTX-321. For more information about the Phase 1 clinical trial of RTX-321, please visit clinicaltrials.gov (NCT04672980).

About RTX-321
RTX-321 is an allogeneic, off-the-shelf aAPC therapy product candidate that is engineered to induce a tumor-specific immune response by expanding antigen-specific T cells. RTX-321 expresses hundreds of thousands of copies of an HPV peptide antigen bound to major histocompatibility complex (MHC) class I proteins, the costimulatory molecule 4-1BBL and the cytokine IL-12 on the cell surface to mimic human T cell-APC interactions.

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 recently named among the Top Places to Work in Massachusetts by the Boston Globe, and its 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 pipeline of Red Cell Therapeutics, including RTX-321 for the treatment of HPV 16-positive cancers, the potential therapeutic benefits of RTX-321, timelines related to the Phase 1 clinical

trial of RTX-321, our expectations regarding our ability to expand manufacturing capabilities, our expectations regarding the potential shelf life of our frozen drug substance for RTX-321, our expectations regarding 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 Red Cell Therapeutic product candidates and their therapeutic potential and other risks identified in our SEC filings, including including our Annual Report on Form 10-K for the year ended December 31, 2020, and subsequent filings with the SEC and risks and uncertainties related to the severity and duration of the impact of COVID-19 on our business and operations. 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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