Caribou Biosciences Announces the FDA Granted Regenerative Medicine Advanced Therapy (RMAT) and Fast Track Designations to CB-010, an Allogeneic Anti-CD19 CAR-T Cell Therapy

November 29, 2022

-- RMAT and Fast Track designations follow CB-010's durable complete responses at dose level 1 in the ANTLER Phase 1 trial --

-- CB-010 is the 1st allogeneic CAR-T cell therapy in the clinic with a PD-1 knockout --

BERKELEY, CA, Nov. 29, 2022 (GLOBE NEWSWIRE) -- Caribou Biosciences, Inc. (Nasdaq: CRBU), a leading clinical-stage CRISPR genome-editing biopharmaceutical company, today announced that the U.S. Food and Drug Administration (FDA) has granted CB-010 Regenerative Medicine Advanced Therapy (RMAT) designation for relapsed or refractory large B cell lymphoma (LBCL) and Fast Track designation for relapsed or refractory B cell non-Hodgkin lymphoma (r/r B-NHL). CB-010, an allogeneic anti-CD19 CAR-T cell therapy with a PD-1 knockout, is being evaluated in the company's ongoing ANTLER Phase 1 clinical trial in patients with r/r B-NHL, which can enroll three LBCL subtypes: diffuse large B cell lymphoma (DLBCL), primary mediastinal large B cell lymphoma (PMBCL), and high-grade B cell lymphoma (HGBL). CB-010 is the first allogeneic anti-CD19 CAR-T cell therapy in the clinic with a PD-1 knockout, a genome-editing strategy designed to improve the persistence of antitumor activity by limiting premature CAR-T cell exhaustion.

"RMAT and Fast Track designations for CB-010 are important recognitions of the significant unmet patient need for an off-the-shelf cell therapy in the treatment of aggressive B-NHL," said Rachel Haurwitz, Ph.D., Caribou's president and chief executive officer. "Through genome editing with our precision CRISPR chRDNA genome-editing technology, CB-010 has been designed with a PD-1 knockout strategy to improve the persistence of antitumor activity by limiting premature CAR-T cell exhaustion. In our ANTLER Phase 1 trial, 3 of 6 patients treated with CB-010 at dose level 1 maintained a durable complete response at 6 months. We are encouraged that CB-010 has demonstrated early potential as an off-the-shelf cell therapy that may meaningfully rival autologous cell therapies."

Encouraging safety data and antitumor activity for CB-010 at dose level 1 ($40x10^6$ CAR-T cells) have been reported from the ANTLER trial. As presented at the European Hematology Association (EHA) 2022 Congress, 6 of 6 patients (100%) achieved a complete response (CR) as best response after treatment with CB-010 at dose level 1 ($40x10^6$ CAR-T cells). Subsequently, at 6 months, 3 of 6 patients (50%) maintained a CR. Fifteen months is the longest CR maintained to date, observed in the first patient dosed in the ANTLER trial. CB-010 was generally well tolerated at dose level 1.

Both RMAT and Fast Track designations are dedicated programs designed to expedite the development and review processes for promising therapeutic candidates intended to address an unmet medical need in patients with serious conditions. These designations provide important benefits in the drug development process and are designed to facilitate and expedite development and regulatory review, including providing eligibility for Priority and Rolling Reviews, and Accelerated Approval, if relevant criteria are satisfied.

About CB-010
CB-010 is the lead product candidate from Caribou's allogeneic CAR-T cell therapy platform and is being evaluated in patients with relapsed or refractory B cell non-Hodgkin lymphoma (r/r B-NHL) in the ongoing ANTLER Phase 1 trial. CB-010 is an allogeneic anti-CD19 CAR-T cell therapy engineered using Cas9 CRISPR hybrid RNA-DNA (chRDNA) technology to insert a CD19-specific CAR into the TRAC gene and knock out PD-1 to boost the persistence of antitumor activity. CB-010 is the first allogeneic CAR-T cell therapy in the clinic with a PD-1 knockout. CB-010 has been granted Regenerative Medicine Advanced Therapy (RMAT), Fast Track, and Orphan Drug designations. Additional information on the ANTLER trial can be found at https://clinicaltrials.gov/ using identifier NCT04637763.

About Caribou's Novel Next-Generation CRISPR Platform
CRISPR genome editing uses easily designed, modular biological tools to make DNA changes in living cells. There are two basic components of Class 2 CRISPR systems: the nuclease protein that cuts DNA and the RNA molecule(s) that guide the nuclease to generate a site-specific, double-stranded break, leading to an edit at the targeted genomic site. CRISPR systems are capable of editing unintended genomic sites, known as off-target editing, which may lead to harmful effects on cellular function and phenotype. In response to this challenge, Caribou has developed CRISPR hybrid RNA-DNA guides (chRDNAs; pronounced "chardonnays") that direct substantially more precise genome editing compared to all-RNA guides. Caribou is deploying the power of its Cas12a chRDNA technology to carry out multiple edits at high efficiency, including multiplex gene insertions, to develop CRISPR-edited therapies.

About Caribou Biosciences, Inc.
Caribou Biosciences is a clinical-stage CRISPR genome-editing biopharmaceutical company dedicated to developing transformative therapies for patients with devastating diseases. The company's genome-editing platform, including its proprietary Cas12a chRDNA technology, enables superior precision to develop cell therapies that are specifically engineered for enhanced persistence. Caribou is advancing a pipeline of off-the-shelf CAR-T and CAR-NK cell therapies for the treatment of patients with hematologic malignancies and solid tumors.

For more information about Caribou, visit www.cariboubio.com and follow the company @CaribouBio.

"Caribou Biosciences" and the Caribou logo are registered trademarks of Caribou Biosciences, Inc.

Forward-Looking Statements
This press release contains forward-looking statements, within the meaning of the Private Securities Litigation Reform Act of 1995. These forward-looking statements include, without limitation, statements related to Caribou's strategy, plans, and objectives, and expectations regarding its clinical and preclinical development programs, including its timing and expectations relating to the release of patient data from its ongoing ANTLER Phase 1 clinical trial for CB-010, as well as the timing of regulatory review and approval. Management believes that these forward-looking statements are reasonable as and when made. However, such forward-looking statements are subject to risks and uncertainties, and actual results may differ materially from any future results expressed or implied by the forward-looking statements. Risks and uncertainties include, without limitation, risks inherent in development of cell therapy products; uncertainties related to the initiation, cost, timing, progress, and results of current and future research and development programs, preclinical studies, and clinical trials; and the risk that initial or interim clinical trial data will not ultimately be predictive of the safety and efficacy of Caribou's product candidates or that clinical outcomes may differ as more patient data becomes available; as well as other

risk factors described from time to time in Caribou's filings with the Securities and Exchange Commission, including its Annual Report on Form 10-K for the year ended December 31, 2021 and subsequent filings. In light of the significant uncertainties in these forward-looking statements, you should not rely upon forward-looking statements as predictions of future events. Except as required by law, Caribou undertakes no obligation to update publicly any forward-looking statements for any reason.

Caribou Biosciences, Inc. Contacts:
Investors:
Amy Figueroa, CFA
afigueroa@caribioubio.com

Media:
Peggy Vorwald, Ph.D.
pvorwald@caribioubio.com

Investors and Media:
Elizabeth Wolffe, Ph.D., and Sylvia Wheeler
Wheelhouse LSA
lwolffe@wheelhouselsa.com
swheeler@wheelhouselsa.com