Caribou Biosciences Announces FDA Granted Fast Track Designation to CB-011, an Allogeneic CAR-T Cell Therapy for Relapsed or Refractory Multiple Myeloma

April 4, 2023

-- CaMMouflage Phase 1 trial for CB-011 enrolling patients with r/r MM at dose level 1 --

BERKELEY, Calif., April 04, 2023 (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 Fast Track designation to CB-011, which is being developed for relapsed or refractory multiple myeloma (r/r MM). CB-011 is being evaluated in the company's ongoing CaMMouflage Phase 1 clinical trial in patients with r/r MM.

"Fast Track designation for CB-011 allows us instrumental interactions with the FDA as we progress our clinical development and regulatory plans for CB-011. This designation could not be more timely as we recently dosed our first patient in the CaMMouflage Phase 1 trial," said Syed Rizvi, MD, Caribou's chief medical officer. "Our goal is to develop CB-011 as a readily available off-the-shelf treatment option for patients with relapsed or refractory multiple myeloma to overcome the need for apheresis or bridging therapy, variable quality and long manufacturing timelines, manufacturing failures, or the inability to bear the burden of treatments that require frequent dosing over several months."

Fast Track designation is designed to expedite the development and review processes for promising therapeutic candidates that may fill an unmet medical need. Clinical programs with Fast Track designation may benefit from early and frequent communication with the FDA throughout the regulatory review process and may also be eligible for Accelerated Approval and Priority Review if relevant criteria are met.

About CB-011
CB-011 is the second product candidate from Caribou's allogeneic CAR-T cell therapy platform and is being evaluated in patients with relapsed or refractory multiple myeloma (r/r MM) in the CaMMouflage Phase 1 trial. CB-011 is an allogeneic anti-BCMA CAR-T cell therapy engineered using Cas12a chRDNA technology. CB-011 is the first allogeneic CAR-T cell therapy in the clinic, to Caribou's knowledge, that is engineered to improve antitumor activity through an immune cloaking strategy with a *B2M* knockout and insertion of a B2M–HLA-E fusion protein to blunt immune-mediated rejection. Additional information on the CaMMouflage trial (NCT05722418) can be found at clinicaltrials.gov.

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 have exhibited editing at 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 high efficiency multiple edits, 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 armored to potentially improve antitumor activity. Caribou is advancing a pipeline of off-the-shelf cell therapies from its CAR-T and CAR-NK platforms as readily available treatments for patients with hematologic malignancies and solid tumors.

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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 expectations relating to the enrollment, status, and updates from its CaMMouflage Phase 1 clinical trial for CB-011, 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 the development of cell therapy products; uncertainties related to the initiation, cost, timing, progress, and results of Caribou's 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; the risk that preclinical study results we observed will not be borne out in human patients; 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, 2022 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.

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