Caribou Biosciences Announces the FDA has Granted Fast Track Designations to CB-010 in Refractory SLE and to CB-012 in Relapsed or Refractory AML

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-- Fast Track designation is designed to expedite clinical development and regulatory review timelines --

-- Additional FDA designation for CB-010 following earlier RMAT and Fast Track designations in B-NHL --

-- CB-010 GALLOP Phase 1 trial in patients with LN and ERL is on track to initiate by YE 2024 --

-- CB-012 AMpLify Phase 1 trial advancing in dose escalation --

BERKELEY, Calif., Sept. 03, 2024 (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) granted Fast Track designations to CB-010 for refractory systemic lupus erythematosus (SLE) and to CB-012 for relapsed or refractory acute myeloid leukemia (r/r AML). CB-010, an allogeneic anti-CD19 CAR-T cell therapy, will be evaluated in the GALLOP Phase 1 clinical trial in patients with lupus nephritis (LN) and extrarenal lupus (ERL), subcategories of SLE. The GALLOP clinical trial is on track to initiate by year-end 2024. CB-012, an allogeneic anti-CLL-1 CAR-T cell therapy, is being evaluated in the company's ongoing AMpLify Phase 1 clinical trial in patients with r/r AML.

"We are pleased to receive Fast Track designations from the FDA. This is a testament to the significant unmet need and the potential of CB-010 and CB-012 as readily available, off-the-shelf CAR-T cell therapies," said Tina Albertson, MD, PhD, Caribou's chief medical officer. "Based on previously reported data in our ongoing trial for patients with relapsed or refractory B cell non-Hodgkin lymphoma, CB-010 has shown encouraging safety, efficacy, and prolonged B cell aplasia following a single dose. Based on these data, we are eager to expand the clinical development program for CB-010 into LN and ERL, prevalent and severe autoimmune diseases."

Dr. Albertson continued, "Most patients with acute AML are refractory or relapse quickly after currently available therapeutic options, and allogeneic hematopoietic stem cell transplant is the only potentially curative option after salvage chemotherapy regimens. As we advance CB-012 and enroll patients at dose level 2, we are focused on our goal to deliver an effective, off-the-shelf treatment option for patients living with this disease."

Fast Track designation is intended to help rapidly advance the development and review processes for promising therapeutic candidates for serious conditions 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 when relevant criteria are met.

About CB-010
CB-010 is the lead clinical-stage product candidate from Caribou's allogeneic CAR-T cell therapy platform, and it is being evaluated in patients with relapsed or refractory B cell non-Hodgkin lymphoma (r/r B-NHL) in the ongoing ANTLER Phase 1 clinical trial and will be evaluated in patients with lupus nephritis (LN) and extrarenal lupus (ERL) in the GALLOP Phase 1 clinical trial. In the ANTLER clinical trial, Caribou is enrolling second-line (2L) patients with large B cell lymphoma (LBCL) comprised of different subtypes of aggressive r/r B-NHL (DLBCL NOS, PMBCL, HGBL, tFL, and tMZL) who have never received prior CD19-targeted therapy as well as LBCL patients who have relapsed after a prior CD19-targeted therapy. To Caribou's knowledge, CB-010 is the first allogeneic CAR-T cell therapy in the clinic with a PD-1 knockout, a genome-editing strategy designed to improve activity against diseases by limiting premature CAR-T cell exhaustion. CB-010 is also, to Caribou's knowledge, the first anti-CD19 allogeneic CAR-T cell therapy to be evaluated in the 2L LBCL setting. The FDA granted CB-010 Regenerative Medicine Advanced Therapy (RMAT) and Orphan Drug designations for B-NHL and Fast Track designations for both B-NHL and refractory systemic lupus erythematosus (SLE). Additional information on the ANTLER trial (NCT04637763) can be found at clinicaltrials.gov.

About CB-012
CB-012 is a product candidate from Caribou's allogeneic CAR-T cell therapy platform and is being evaluated in the AMpLify Phase 1 clinical trial in patients with relapsed or refractory acute myeloid leukemia (r/r AML). CB-012 is an anti-CLL-1 CAR-T cell therapy engineered with five genome edits, enabled by Caribou's patented next-generation CRISPR technology platform, which uses Cas12a chRDNA genome editing to significantly improve the specificity of genome edits. To Caribou's knowledge, CB-012 is the first allogeneic CAR-T cell therapy with both checkpoint disruption, through a PD-1 knockout, and immune cloaking, through a B2M knockout and B2M–HLA-E fusion protein insertion; both armoring strategies are designed to improve antitumor activity. Caribou has exclusively in-licensed from Memorial Sloan Kettering Cancer Center (MSKCC) in the field of allogeneic CLL-1-targeted cell therapy a panel of fully human scFvs targeting CLL-1, from which the company has selected a scFv for the generation of the company's CAR. CB-012 was granted Fast Track designation by the FDA. Additional information on the AMpLify trial (NCT06128044) 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 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 chRDNA technology to carry out high efficiency multiple edits, 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 Cas12a chRDNA technology, enables superior precision to develop cell therapies that are armored to potentially improve activity against disease. Caribou is advancing a pipeline of off-the-shelf cell therapies from its CAR-T platform as readily available treatments for patients with hematologic malignancies and autoimmune diseases. Follow us @CaribouBio and visit www.cariboubio.com.

Forward-Looking Statements
This press release contains forward-looking statements within the meaning of the Private Securities Litigation Reform Act of 1995. In some cases, you

can identify forward-looking statements by terms such as “may,” “will,” “should,” “expect,” “plan,” “anticipate,” “could,” “intend,” “target,” “project,” “contemplate,” “believe,” “estimate,” “predict,” “potential,” or “continue,” or the negative of these terms or other similar expressions, although not all forward-looking statements contain these words. These forward-looking statements include, without limitation, statements related to Caribou’s strategy, plans, and objectives, and expectations regarding its clinical development programs, including its expectations relating to development, regulatory approval, results, and the timing of and updates from its ANTLER Phase 1 clinical trial for CB-010, its GALLOP Phase 1 clinical trial for CB-010, and its AMpLify Phase 1 clinical trial for CB-012. 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, preliminary, 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 patient enrollment continues and as more patient data becomes available; the risk that preclinical study results observed will not be borne out in human patients or different conclusions or considerations are reached once additional data have been received and fully evaluated; the ability to obtain key regulatory input and approvals; 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, 2023 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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