Caribou Biosciences Presents New Preclinical Data on CB-012, an Allogeneic Anti-CLL-1 CAR-T Cell Therapy, at the 2023 AACR Annual Meeting

April 17, 2023

-- CB-012 genome-edited armoring strategy significantly reduced tumor burden and improved overall survival in AML xenograft models --

-- CB-012 IND-enabling studies ongoing; IND submission in r/r AML planned for H2 2023 --

BERKELEY, Calif., April 17, 2023 (GLOBE NEWSWIRE) -- Caribou Biosciences, Inc. (Nasdaq: CRBU), a leading clinical-stage CRISPR genome-editing biopharmaceutical company, today presents a poster of preclinical data demonstrating the promise of CB-012, a next-generation CRISPR-edited allogeneic anti-CLL-1 CAR-T cell therapy, as a therapeutic candidate for adult patients with relapsed or refractory acute myeloid leukemia (r/r AML). The presentation takes place at the 2023 American Association for Cancer Research (AACR) Annual Meeting today from 1:30 pm to 5:00 pm EDT at the Orange County Convention Center, Orlando, Florida.

“CLL-1 is a compelling target for AML because it is highly expressed on myeloid cancer cells and is enriched on leukemic stem cells, but it is not expressed on hematopoietic stem cells,” said Steve Kanner, PhD, Caribou’s chief scientific officer. “CB-012 is engineered with 5 edits, using our Cas12a chRDNA technology, which armor the CAR-T cells for improved antitumor activity through checkpoint disruption and immune cloaking. The preclinical data in the poster demonstrate that CB-012 exhibits enhanced antitumor activity against established AML xenografts.”

Caribou’s patented next-generation CRISPR Cas12a chRDNA genome-editing technology platform, which maintains high genomic integrity and significantly improves the specificity of genome edits, was used to engineer the 5 genome edits implemented in the manufacture of CB-012. CB-012 is the first allogeneic CAR-T cell therapy, to Caribou’s knowledge, with both checkpoint disruption, through a PD-1 knockout (KO), and immune cloaking, through a *B2M* KO and B2M–HLA-E fusion transgene insertion. These armoring strategies were designed to promote the durability of antitumor activity. Preclinical data presented at the AACR meeting show:

- CB-012 targets, becomes activated, proliferates, and demonstrates antitumor activity against a broad panel of AML cancer cell lines
- Immune cloaking protects CB-012 from NK cell-mediated cytotoxicity
- Mice harbouring AML xenograft models treated with CB-012 having a PD-1 KO showed extended survival relative to mice injected with control CAR-T cells that express PD-1 and that only contain 4 out of the 5 edits
- CB-012 demonstrated significant antitumor efficacy and prolonged survival in AML xenograft models

“The preclinical data presented at AACR further support the clinical development of CB-012, Caribou’s third program from our allogeneic CAR-T cell therapy platform,” said Rachel Haurwitz, PhD, Caribou’s president and chief executive officer. “Preclinical studies supporting our planned IND submission for CB-012 in relapsed or refractory AML are advancing and we are on track to submit our IND to the FDA in the second half of this year.”

Details of the poster presentation are below and the full poster can be found on Caribou’s website under Scientific Publications.

Title: CB-012, an allogeneic anti-CLL-1 CAR-T cell therapy engineered with next-generation CRISPR technology to resist both the immunosuppressive tumor microenvironment and immune cell-mediated rejection, for patients with relapsed or refractory acute myeloid leukemia
Presenter: Tristan Fowler, PhD, associate director of preclinical pharmacology, Caribou Biosciences
Session: PO.CL07.04 - Adoptive Cell Therapy 2
Session Date: Monday, April 17, 2023
Presentation Time: 1:30 - 5:00 pm EDT
Location: Orange County Convention Center, section 37
Abstract number: 3201

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 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 expectations relating to the submission of its IND application for CB-012 as well as additional updates and results from its IND-enabling preclinical studies. 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, including CB-012; 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, including those for CB-012; and the risk that future initial or interim clinical trial data will not ultimately be predictive of the safety and efficacy of Caribou's product candidates, including CB-012 or that clinical outcomes may differ as more patient data becomes available; the risk that preclinical study results, including those for CB-012 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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