



Caribou Biosciences Presents Data on Mechanism Underlying Superior Specificity of Caribou's chRDNA Genome-Editing Technology in Primary Human T cells

May 16, 2022

-- Data presented at the 25th Annual Meeting of the American Society for Gene and Cell Therapy (ASGCT) --

BERKELEY, Calif., May 16, 2022 (GLOBE NEWSWIRE) -- [Caribou Biosciences, Inc.](#) (Nasdaq: CRBU), a leading clinical-stage CRISPR genome-editing biopharmaceutical company, today announced the presentation of studies in primary human T cells highlighting the mechanism underlying the superior specificity of the company's CRISPR hybrid RNA-DNA (chRDNA) guides for genome editing.

"Our chRDNA guides provide a robust and reproducible approach for maximizing the specificity of CRISPR genome editing, which is key to improving the persistence and potential efficacy of our allogeneic cell therapies," said Steve Kanner, Ph.D., Caribou's chief scientific officer. "Through simple iterative engineering of DNA positions in our chRDNA guides, we optimize the activity and specificity of editing at different sites in the genome."

In studies with primary human T cells, optimized chRDNA guides were rapidly generated to provide higher specificity editing compared to either all-RNA guides or when employing an engineered high fidelity Cas9 variant. These high specificity chRDNA guides can be used with either the Cas9 protein or the Cas12a protein. The presentation highlights the underlying mechanism of this enhanced specificity by comparing the structure of Cas9 nuclease with all RNA guides compared with chRDNA guides. The data show differences between the structural conformation of the complex when targeting the intended specific site in the chromosome versus unintended "off-target" sites, providing an explanation for the improved specificity. Together, these data demonstrate that chRDNA guides enable highly efficient and precise genome editing, emphasizing their potential superiority for use in therapeutic applications.

The data are being presented virtually today at the 25th Annual Meeting of the American Society for Gene and Cell Therapy (ASGCT) which is being held May 16-19, 2022 in Washington, D.C. The presentation, titled "*Conformational Control of Cas Endonucleases by CRISPR Hybrid RNA-DNA Guides Mitigates Off-Target Activity in T Cells*" is available on the [Presentations](#) page of the Investors section of the Caribou website.

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 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 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.

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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. 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 CRISPR technologies and cell therapy products; and 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; 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:

Amy Figueroa, CFA
Investor Relations and Corporate
Communications
afigueroa@cariboubio.com

Investors and Media:

Elizabeth Wolffe, Ph.D., and Sylvia Wheeler

Wheelhouse LSA

lwolfe@wheelhousesa.com

swheeler@wheelhousesa.com