Caribou Biosciences Reports Second Quarter 2024 Financial Results and Provides Business Update

August 6, 2024

-- Advancing four clinical-stage programs for hematologic malignancies and autoimmune diseases; clinical data reports planned for 2024 and H1 2025 --

-- Enrolling 2L LBCL and prior CD19 relapsed LBCL patients based on CB-010 ANTLER Phase 1 data presented at 2024 ASCO Annual Meeting; data to be presented H1 2025 --

-- $311.8 million in cash, cash equivalents, and marketable securities expected to fund the current operating plan into H2 2026; cash runway extension of at least 6 months versus prior guidance --

BERKELEY, Calif., Aug. 06, 2024 (GLOBE NEWSWIRE) -- Caribou Biosciences, Inc. (Nasdaq: CRBU), a leading clinical-stage CRISPR genome-editing biopharmaceutical company, today reported financial results for the second quarter 2024 and reviewed recent pipeline progress.

"We are advancing our lead off-the-shelf CAR-T cell therapy, CB-010, in the ANTLER Phase 1 trial with a partial HLA matching strategy with the objective of developing an allogeneic CAR-T cell therapy that can meaningfully rival the autologous CAR-T cell therapies," said Rachel Haurwitz, PhD, Caribou's president and chief executive officer. "We are enrolling approximately 20 second-line and 10 prior CD19 relapsed LBCL patients, and we plan to present initial data for both patient cohorts in the first half of 2025. For CB-011, we expect to report initial dose escalation data in patients with relapsed or refractory multiple myeloma by the end of this year. For CB-012, dose level 1 was cleared, and we are enrolling patients at dose level 2 in the AMpLify Phase 1 trial. We continue to focus our efforts and resources on rapidly advancing our four oncology and autoimmune disease clinical-stage programs through multiple clinical data milestones expected in 2024 and 2025."

Clinical highlights
CB-010, a clinical-stage allogeneic anti-CD19 CAR-T cell therapy for B cell non-Hodgkin lymphoma

- In June 2024, Caribou presented clinical data from the ongoing ANTLER Phase 1 clinical trial that indicate a single dose of CB-010 has the potential to rival the safety and efficacy of approved autologous CAR-T cell therapies. The clinical results were presented during a poster presentation at the 2024 American Society of Clinical Oncology (ASCO) Annual Meeting.
- At ASCO, Caribou presented data on the first 46 patients enrolled in ANTLER. Three dose levels of CB-010 were evaluated ($40x10^6$, $80x10^6$, and $120x10^6$ CAR-T cells) and $80x10^6$ CAR-T cells was selected as the recommended Phase 2 dose (RP2D). In dose escalation, 16 patients with multiple subtypes of aggressive relapsed or refractory B cell non-Hodgkin lymphoma (r/r B-NHL) were enrolled, and, in dose expansion, 30 patients with second-line large B cell lymphoma (2L LBCL) were enrolled. As of the April 1, 2024 data cutoff date, results demonstrated:
    - CB-010 was generally well tolerated. No Grade 3 or higher cytokine release syndrome (CRS) and no graft-versus-host disease (GvHD) was observed.
    - A retrospective analysis of all patient data demonstrated that patients who received a dose of CB-010 manufactured from a healthy donor who shared four or more matching human leukocyte antigen ("HLA") alleles with the patient (referred to as partial HLA matching) showed the potential for improved efficacy.
    - Pharmacokinetic (PK) data showed that partial HLA matching correlated with increased CAR-T cell expansion and persistence. Pharmacodynamic (PD) data showed extended B cell aplasia and rapid recovery of patients' endogenous T and NK cells.
- Based on these data, Caribou has begun dosing a cohort of approximately 20 2L LBCL patients to prospectively confirm that partial HLA matching may improve patient outcomes.
- Caribou also is enrolling a cohort of up to 10 patients who have relapsed following any prior CD19-targeted therapy in a proof-of-concept cohort in this population of unmet need. This cohort will also incorporate partial HLA matching between donors and patients.
- Caribou plans to initiate a pivotal Phase 3 trial in the second half of 2025, should data confirm improved outcomes for patients receiving a partially HLA matched dose of CB-010 and following agreement with the FDA on a pivotal trial design.

CB-010, a clinical-stage allogeneic anti-CD19 CAR-T cell therapy for lupus

- Caribou plans to initiate the GALLOP Phase 1 clinical trial to evaluate a single infusion of CB-010 in adult patients with lupus nephritis (LN) and extrarenal lupus (ERL). The trial will incorporate partial HLA matching between donors and patients.
- Caribou plans to initiate the GALLOP Phase 1 clinical trial in adult patients with LN and ERL by year-end 2024.

CB-011, a clinical-stage allogeneic anti-BCMA CAR-T cell therapy for multiple myeloma

- Caribou is enrolling patients with relapsed or refractory multiple myeloma (r/r MM) in the dose escalation portion of the ongoing CaMMouflage Phase 1 clinical trial.
- Caribou plans to present initial dose escalation data from the ongoing CaMMouflage Phase 1 clinical trial by year-end 2024.

CB-012, a clinical-stage allogeneic anti-CLL-1 CAR-T cell therapy for acute myeloid leukemia

- In June 2024, a poster was presented at ASCO on the AMpLify Phase 1 trial design for CB-012 in adults with relapsed or refractory acute myeloid leukemia (r/r AML).
- Caribou is enrolling patients with r/r AML in the dose escalation portion of the ongoing AMpLify Phase 1 clinical trial. Enrollment has concluded for dose level 1 ($25x10^6$ CAR-T cells, N=3) and patients are being enrolled at dose level 2 ($75x10^6$ CAR-T cells).

Corporate updates
Appointed autoimmune expert to Caribou's scientific advisory board

- In July 2024, Terri Laufer, MD, was appointed to Caribou's scientific advisory board. Dr. Laufer is a leading rheumatologist known for her extensive research into immune cell regulation and dysfunction that leads to autoimmune diseases. She is an emeritus associate professor of medicine at the Perelman School of Medicine at the University of Pennsylvania and an attending rheumatologist at the Penn Presbyterian Medical Center and Philadelphia VA Medical Center.

Extended cash runway into H2 2026

- In July 2024, Caribou discontinued the preclinical research activities associated with its allogeneic CAR-NK platform and reduced its workforce by approximately 12%. The workforce reduction, together with other cost containment measures, are expected to extend the cash runway by at least 6 months, into H2 2026. The Company will incur approximately $0.5 million to $1.0 million in one-time costs consisting primarily of cash severance costs, benefits, and transition support services for impacted employees.

Anticipated milestones

- **CB-010 ANTLER:** Caribou plans to present initial data from both the additional HLA-matched 2L and prior CD19 relapsed LBCL patient cohorts in H1 2025. Caribou plans to initiate a pivotal Phase 3 clinical trial in H2 2025 should data confirm improved outcomes for patients receiving a partially HLA matched dose of CB-010.
- **CB-010 GALLOP:** Caribou plans to initiate the GALLOP Phase 1 clinical trial in adult patients with LN and ERL by year-end 2024.
- **CB-011 CaMMouflage:** Caribou plans to present initial dose escalation data from the ongoing CaMMouflage Phase 1 clinical trial by year-end 2024.
- **CB-012 AMpLify:** Caribou plans to provide updates on dose escalation as the AMpLify Phase 1 clinical trial in r/r AML advances.

Second quarter 2024 financial results
Cash, cash equivalents, and marketable securities: Caribou had $311.8 million in cash, cash equivalents, and marketable securities as of June 30, 2024, compared to $372.4 million as of December 31, 2023. Caribou expects these cash, cash equivalents, and marketable securities will be sufficient to fund its current operating plan into H2 2026.

Licensing and collaboration revenue: Revenue from Caribou's licensing and collaboration agreements was $3.5 million for the three months ended June 30, 2024, compared to $3.8 million for the same period in 2023. The decrease was primarily due to the now-terminated AbbVie Collaboration and License Agreement, partially offset by an increase in revenue recognized under the Information Rights Agreement Caribou entered into with Pfizer on June 29, 2023. Licensing and collaboration revenue for the three months ended June 30, 2024, includes $1.6 million in a one-time receipt of non-cash equity consideration from one of Caribou's licensees.

R&D expenses: Research and development expenses were $35.5 million for the three months ended June 30, 2024, compared to $26.5 million for the same period in 2023. The increase was primarily due to costs to advance pipeline programs, including the CB-010 ANTLER, CB-011 CaMMouflage, and CB-012 AMpLify Phase 1 clinical trials; personnel-related expenses, including stock-based compensation, due to headcount increases; and facilities and other allocated expenses.

G&A expenses: General and administrative expenses were $11.5 million for the three months ended June 30, 2024, compared to $10.1 million for the same period in 2023. The increase was primarily due to personnel-related expenses, including stock-based compensation, due to headcount increases, and legal expenses and other service-related expenses. These increases were partially offset by a decrease in patent prosecution and maintenance fees.

Net loss: Caribou reported a net loss of $37.7 million for the three months ended June 30, 2024, compared to $29.5 million for the same period in 2023.

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 on 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 and, for r/r B-NHL, CB-010 has been granted Regenerative Medicine Advanced Therapy (RMAT), Fast Track, and Orphan Drug designations by the FDA. Additional information on the ANTLER trial (NCT04637763) can be found at clinicaltrials.gov.

About CB-011
CB-011 is a 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 genome-editing technology. To Caribou's knowledge, CB-011 is the first allogeneic CAR-T cell therapy in the clinic 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. CB-011 has been granted Fast Track and orphan drug designations by the FDA. Additional information on the CaMMouflage trial (NCT05722418) 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. 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 diseases. Caribou is advancing a pipeline of clinical-stage off-the-shelf cell therapies from its CAR-T cell platform as readily available treatments for patients with hematologic malignancies and autoimmune diseases. Follow us @CaribouBio and visit www.caribou bio.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 and preclinical development programs, including its expectations relating to (i) the timing of reporting additional dose expansion data in its ANTLER Phase 1 clinical trial for CB-010, including data from both additional HLA-matched 2L LBCL and prior CD19 cohorts, and the timing of an ANTLER pivotal Phase 3 clinical trial; (ii) the timing of and updates from its CaMMouflage Phase 1 clinical trial for CB-011 and expectations regarding the timing of presenting the initial dose escalation data; (iii) the timing of and updates from its AMpLify Phase 1 clinical trial for CB-012; (iv) the timing of and updates from its GALLOP Phase 1 clinical trial for CB-010 in patients with LN and ERL; (v) the anticipated costs associated with the workforce reduction, including specific categories of costs and future cash expenditures and the timing of when the reduction is expected to be completed and the anticipated costs recognized; and (vi) its expected funding runway of cash, cash equivalents, and marketable securities. 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.

Caution should be exercised when interpreting results from separate trials involving other CAR-T cell therapies. The results of other CAR-T cell therapies presented or referenced in this press release have been derived from publicly available reports of clinical trials not conducted by Caribou, and Caribou has not performed any head-to-head trials comparing any of these other CAR-T cell therapies with CB-010. As such, the results of these other clinical trials may not be comparable to clinical results for CB-010. The design of these other clinical trials varies in material ways from the design of the ANTLER clinical trial for CB-010, including with respect to patient populations, follow-up times, clinical trial phases, and subject characteristics. As a result, cross-trial comparisons may have no interpretive value on Caribou's existing or future clinical results. For further information and to understand these material differences, you should read the reports for the other CAR-T cell therapy clinical trials and the sources included in Caribou's corporate presentations on its website.

Caribou Biosciences, Inc.
Condensed Consolidated Balance Sheet Data
(in thousands)
(unaudited)

	June 30, 2024	December 31, 2023
Cash, cash equivalents, and marketable securities	$ 311,773	$ 372,404
Total assets	372,938	432,209
Total liabilities	62,474	63,808
Total stockholders' equity	310,464	368,401
Total liabilities and stockholders' equity	$ 372,938	$ 432,209

Caribou Biosciences, Inc.
Condensed Consolidated Statement of Operations
(in thousands, except share and per share data)
(unaudited)

	Three Months Ended June 30,		Six Months Ended June 30,	
	2024	2023	2024	2023
Licensing and collaboration revenue	$ 3,464	$ 3,755	$ 5,893	$ 7,257
Operating expenses:				
Research and development	35,480	26,503	69,268	52,212
General and administrative	11,485	10,120	26,128	19,029
Total operating expenses	46,965	36,623	95,396	71,241
Loss from operations	(43,501)	(32,868)	(89,503)	(63,984)
Other income (expense):				
Change in fair value of equity securities	(102)	22	(102)	7
Change in fair value of the MSKCC success payments liability	1,795	279	2,098	534
Other income - net	4,111	3,048	8,576	5,880
Total other income (expense)	5,804	3,349	10,572	6,421
Net loss	(37,697)	(29,519)	(78,931)	(57,563)
Other comprehensive (loss) income:				
Net unrealized (loss) gain on available-for-sale marketable securities, net of tax	3	(406)	(349)	382
Net comprehensive loss	$ (37,694)	$ (29,925)	$ (79,280)	$ (57,181)
Net loss per share, basic and diluted	$ (0.42)	$ (0.48)	$ (0.88)	$ (0.94)
Weighted-average common shares outstanding, basic and diluted	90,340,932	61,417,934	89,821,935	61,302,863

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