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

November 6, 2024

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

-- Enrolling patients with LBCL (2L and after prior CD19-targeted therapies) with HLA matching strategy in CB-010 ANTLER Phase 1 trial; next data report planned for H1 2025 --

-- Continuing dose escalation portion of the CB-011 CaMMouflage Phase 1 trial in r/r MM with higher lymphodepletion regimen following observations of efficacy; dose escalation data to be presented H1 2025 --

-- $281.0 million in cash, cash equivalents, and marketable securities expected to fund the current operating plan into H2 2026 --

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

"We are focused on rapidly advancing our four clinical-stage programs for oncology and autoimmune diseases toward multiple data milestones expected in 2025," said Rachel Haurwitz, PhD, Caribou's president and chief executive officer. "To highlight key progress this quarter, in the CB-011 CaMMouflage Phase 1 trial, we are encouraged by observations of efficacy with the implementation of a higher lymphodepletion regimen. We continue to enroll more patients with relapsed or refractory multiple myeloma and plan to report dose escalation data in the first half of 2025. In addition, we continue to expand the development of our lead program CB-010 into lupus and activation activities at multiple sites are underway as we plan to initiate our Phase 1 GALLOP trial by the end of this year."

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

- Caribou is enrolling approximately 20 additional second-line large B cell lymphoma (2L LBCL) patients in the ongoing ANTLER Phase 1 clinical trial. Caribou is enrolling these patients to confirm the progression-free survival (PFS) trend observed previously with CB-010 product partially matched to the patient for human leukocyte antigens (HLA).
- Caribou is also 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 be partially matched for HLA with the product donor.
- Caribou plans to initiate a pivotal Phase 3 trial of CB-010 in the second half of 2025, should data from the additional 2L LBCL patients confirm the initial observation that partial HLA matching of patient to the donor is associated with improved outcomes. The Phase 3 trial would be initiated after agreement with the FDA on a pivotal trial design.

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

- In September 2024, the U.S. Food and Drug Administration (FDA) granted Fast Track designation to CB-010 for refractory systemic lupus erythematosus (SLE).
- An abstract was accepted for a poster presentation at the American College of Rheumatology Convergence 2024 in Washington, DC.
    - The poster titled "Preclinical Analysis of CB-010, an Allogeneic anti-CD19 CAR-T Cell Therapy with a PD-1 Knockout, for the Treatment of Patients with Refractory Systemic Lupus Erythematosus (SLE)" is to be presented by Elizabeth Garner, PhD, Caribou's executive director of T cell therapeutics and translational sciences laboratory, on Saturday, November 16, 2024, 10:30 am-12:30 pm EST.
- 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), subcategories of SLE, by year-end 2024. The trial will incorporate a partial HLA matching strategy.

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

- In the dose escalation portion of the CaMMouflage Phase 1 clinical trial for relapsed or refractory multiple myeloma (r/r MM), Caribou is escalating to dose level 4 ($800x10^6$ CAR-T cells) with a lymphodepletion regimen that includes a higher dose of cyclophosphamide (increased from 300 to 500 mg/m^2/day together with the same fludarabine dose of 30 mg/m^2/day for 3 days) after clearing dose level 3 ($450x10^6$ CAR-T cells) with the same higher lymphodepletion with no dose-limiting toxicities (DLTs).
- Following the observations of efficacy, Caribou is enrolling additional patients as backfill at DL3 with the higher lymphodepletion regimen, which includes 500 mg/m^2/day of cyclophosphamide.
- No DLTs have been observed in CaMMouflage.
- Caribou plans to present dose escalation data on a minimum of 15 patients at active doses from the ongoing CaMMouflage Phase 1 clinical trial in H1 2025.

"We are excited to continue evaluating CB-011 in our CaMMouflage Phase 1 trial," said Caribou's newly appointed chief medical officer, Tina

Albertson, MD, PhD. “Additional patient enrollment with the higher lymphodepletion regimen and longer follow up provide a more robust and meaningful dataset in the first half of 2025 that will inform the future direction of this promising program and its potential for patients with relapsed or refractory multiple myeloma.”

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

- In September 2024, the U.S. FDA granted Fast Track and Orphan Drug designations to CB-012 for 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 2 ($75x10^6$ CAR-T cells, N=3) and no DLTs were observed. Patients are being enrolled at dose level 3 ($150x10^6$ CAR-T cells).

Corporate updates
Appointed Tina Albertson, MD, PhD, as chief medical officer

- In August 2024, Tina Albertson, MD, PhD, was appointed chief medical officer. Dr. Albertson is a highly experienced hematologist and oncologist with a proven track record successfully driving global clinical development of CAR-T cell therapies. Previously, she served as chief medical officer and head of development for Lyell Immunopharma. Earlier, she served as vice president of global drug development at Juno Therapeutics, a Bristol-Myers Squibb company, where she led the global development of BREYANZI (lisocabtagene maraleucel) from IND to filing of the initial BLA that resulted in FDA approval in large B cell lymphoma. At Juno, she led 9 global clinical trials, including 4 registrational trials of BREYANZI in other B cell malignancies and earlier lines of therapy. Dr. Albertson previously served as medical director of clinical development and experimental medicine at Seagen (formerly Seattle Genetics).

Anticipated milestones

- **CB-010 ANTLER:** Caribou plans to present data from both the additional 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 the initial observation that partial HLA matching is associated with improved outcomes for patients.
- **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 dose escalation data from the ongoing CaMMouflage Phase 1 clinical trial in r/r MM in H1 2025.
- **CB-012 AMpLify:** Caribou plans to provide updates on dose escalation as the AMpLify Phase 1 clinical trial in r/r AML advances.

Third quarter 2024 financial results
Cash, cash equivalents, and marketable securities: Caribou had $281.0 million in cash, cash equivalents, and marketable securities as of September 30, 2024, compared to $372.4 million as of December 31, 2023. Caribou expects its 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 $2.0 million for the three months ended September 30, 2024, compared to $23.7 million for the same period in 2023. The decrease was primarily due to the recognition of deferred revenue in the 2023 period in connection with the termination of the AbbVie Collaboration and License Agreement.

R&D expenses: Research and development expenses were $30.4 million for the three months ended September 30, 2024, compared to $28.6 million for the same period in 2023. The increase was primarily due to costs to advance pipeline programs, including the CB-010 ANTLER, CB-010 GALLOP, CB-011 CaMMouflage, and CB-012 AMpLify Phase 1 clinical trials; personnel-related expenses, including stock-based compensation, primarily due to severance and other related expenses as a result of the July 2024 reduction in workforce; and other research and development expenses to advance preclinical and clinical research, partially offset by a decrease in expenses relating to licenses.

G&A expenses: General and administrative expenses were $9.8 million for the three months ended September 30, 2024, compared to $9.7 million for the same period in 2023. The increase was primarily due to patent prosecution and maintenance costs, partially offset by lower legal and other service-related expenses.

Net loss: Caribou reported a net loss of $34.7 million for the three months ended September 30, 2024, compared to $10.0 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. 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 CAR-T cell activity by limiting premature CAR-T cell exhaustion. 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-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. CB-012 was granted Fast Track and Orphan Drug designations 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.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) plans to present ANTLER clinical trial data from both the additional 2L and prior CD19 relapsed LBCL patient cohorts in H1 2025 and the timing of an ANTLER pivotal Phase 3 clinical trial; (ii) plans to present dose escalation data from the ongoing CaMMouflage Phase 1 clinical trial for CB-011 in r/r MM in H1 2025; (iii) plans to provide updates on dose escalation from the AMpLify Phase 1 clinical trial for CB-012; (iv) the timing of and updates from the GALLOP Phase 1 clinical trial for CB-010 in patients with LN and ERL; and (v) 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.

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

	September 30, 2024	December 31, 2023
Cash, cash equivalents, and marketable securities	$ 281,015	$ 372,404
Total assets	344,334	432,209
Total liabilities	63,131	63,808
Total stockholders' equity	281,203	368,401
Total liabilities and stockholders' equity	$ 344,334	$ 432,209

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

	Three Months Ended September 30,		Nine Months Ended September 30,	
	2024	2023	2024	2023
Licensing and collaboration revenue	$ 2,024	$ 23,662	$ 7,917	$ 30,919
Operating expenses:				

Research and development	30,421	28,584	99,689	80,796
General and administrative	9,841	9,711	35,969	28,740
Total operating expenses	40,262	38,295	135,658	109,536
Loss from operations	(38,238)	(14,633)	(127,741)	(78,617)
Other income:				
Change in fair value of equity securities	(14)	(4)	(116)	3
Change in fair value of the MSKCC success payments liability	(164)	(139)	1,934	395
Other income, net	3,732	4,774	12,308	10,654
Total other income	3,554	4,631	14,126	11,052
Net loss	$ (34,684)	$ (10,002)	$ (113,615)	$ (67,565)
Other comprehensive income:				
Net unrealized gain on available-for-sale marketable securities, net of tax	1,108	155	759	537
Net comprehensive loss	$ (33,576)	$ (9,847)	$ (112,856)	$ (67,028)
Net loss per share, basic and diluted	$ (0.38)	$ (0.12)	$ (1.26)	$ (0.98)
Weighted-average common shares outstanding, basic and diluted	90,455,900	83,783,992	90,034,799	68,878,921

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