Caribou Biosciences Announces Positive Data from CaMMouflage Phase 1 Trial of CB-011 in Multiple Myeloma

November 3, 2025

- *First clinical data disclosure for CB-011 highlights its potential as a best-in-class allogeneic CAR-T cell therapy for relapsed or refractory multiple myeloma*
- *450 million cell dose is the recommended dose for expansion (RDE); dose expansion to initiate by year end and data expected in 2026*
- *92% ORR, 75% ≥CR rate, 91% MRD negativity in the 12-patient, BCMA-naïve cohort treated at the RDE with the selected lymphodepletion regimen*
- *Conference call and webcast scheduled for today at 8:00 am ET*

BERKELEY, Calif., Nov. 03, 2025 (GLOBE NEWSWIRE) -- Caribou Biosciences, Inc. (Nasdaq: CRBU), a leading clinical-stage CRISPR genome-editing biopharmaceutical company, today announced its first clinical data from dose escalation in the ongoing CaMMouflage phase 1 trial evaluating CB-011, an off-the-shelf anti-BCMA CAR-T cell therapy, in relapsed or refractory multiple myeloma (r/r MM), highlighting CB-011 as a potentially best-in-class allogeneic CAR-T cell therapy for this patient population. The Company is advancing the program into dose expansion, which it expects to initiate by the end of this year, and the Company plans to share dose expansion data in 2026.

“Despite a rapidly advancing landscape of treatment options for multiple myeloma, challenges remain with only 10% of eligible patients receiving an autologous CAR-T,” said Adriana Rossi, MD, director of CAR-T and stem cell transplant clinical program at the center of excellence for multiple myeloma at Mount Sinai, and an investigator on the CaMMouflage trial. “I believe the promising responses we are seeing with CB-011 combined with the off-the-shelf nature of this therapy could represent a paradigm shift for patients with relapsed or refractory multiple myeloma. I am excited about the potential this holds for the large number of multiple myeloma patients who simply cannot wait for autologous CAR-T manufacturing and prefer a single dose approach.”

In the dose escalation portion of the CaMMouflage phase 1 clinical trial, safety and efficacy of CB-011 were evaluated in 48 fourth-line and later (4L+) patients at multiple dose levels and following two different lymphodepletion regimens. Thirty-five patients were treated with the selected lymphodepletion (LD) regimen of 500 mg/m^2 cyclophosphamide and 30 mg/m^2 fludarabine daily for three days. A single dose of CB-011 preceded by the selected LD regimen resulted in responses at all dose levels evaluated (150x10^6 [N=6], 300x10^6 [N=13], 450x10^6 [N=13], and 800x10^6 [N=3] CAR-T cells). The 450x10^6 CAR-T cell dose with the selected LD regimen is the recommended dose for expansion (RDE). Twelve BCMA-naïve r/r MM patients were treated with the RDE. The median follow-up for patients dosed with the RDE was 8.3 months, and the longest responding patient is in a stringent complete response (sCR) at 15 months post-infusion. As of the September 24, 2025, data cutoff date, the results for the 12-patient, BCMA-naïve cohort treated with the RDE were as follows:

- 92% (11/12) overall response rate (ORR)
- 75% (9/12) ≥complete response (CR) rate
- 91% (10/11 evaluable patients) achieved minimal residual disease (MRD) negativity (≤10^{-5})
- 7 of 12 patients remained on study as of the data cutoff date in ≥very good partial response (VGPR) 6 months or longer following receipt of a single dose of CB-011

CB-011 had a manageable safety profile across all dose levels and lymphodepletion regimens (N=48), with no cases of graft-versus-host disease (GvHD), immune effector cell-associated enterocolitis (IEC-EC), parkinsonism, or cranial nerve palsies. Treatment emergent adverse events (TEAEs) in ≥25% of all patients treated with CB-011 following the selected LD regimen (N=35) were as follows: neutropenia (80%), anemia (60%), thrombocytopenia (49%), infections (49%), dizziness (31%), cytokine release syndrome (31%), fatigue (31%), leukopenia (29%), decreased appetite (29%), constipation (26%), and pyrexia (26%). Notable adverse events in the RDE cohort included one CB-011-related grade 5 immune effector cell-associated hematotoxicity (ICAHT) on day 90, one grade 5 pneumonia not related to CB-011 on day 50, and one grade 4 CB-011-related Guillain-Barré Syndrome on day 129, which is resolving. In the cohort evaluating the 300x10^6 CAR-T cell dose level following the selected LD regimen, there was one grade 5 respiratory syncytial virus not related to CB-011 on day 73. Prophylactic measures for cytopenias and infections and early intervention for immune effector cell-associated hemophagocytic lymphohistiocytosis-like syndrome (IEC-HS) have been successfully implemented in the protocol.

“We are very encouraged by the compelling results from the CaMMouflage phase 1 trial, which demonstrate that CB-011 is delivering deep, durable responses in high-risk, heavily pretreated multiple myeloma patients with a manageable safety profile. These data establish CB-011’s potential to be a best-in-class allogeneic CAR-T cell therapy that could expand access and bring meaningful benefit to patients who urgently need a readily available, single-dose option,” said Rachel Haurwitz, PhD, Caribou’s president and chief executive officer. “We plan to initiate dose expansion before year end and to report dose expansion data, along with longer follow up on dose escalation data, in 2026.”

Webcast conference call today at 8:00 am ET
Caribou will host a live conference call and webcast Monday, November 3 at 8:00 am ET to discuss the CaMMouflage trial data, as well as the ANTLER phase 1 clinical trial and anticipated pivotal trial design. The presenters will include:

- Adriana Rossi, MD, director of CAR-T and stem cell transplant clinical program at the center of excellence for multiple myeloma at Mt Sinai, and investigator on the CaMMouflage trial

- Mehdi Hamadani, MD, professor of medicine, section chief of hematologic malignancies at Medical College of Wisconsin, and investigator on the ANTLER trial
- Joseph McGuirk, DO, professor of hematology/oncology and division director for hematologic malignancies and cellular therapeutics at University of Kansas Cancer Center
- Rachel Haurwitz, PhD, president and chief executive officer, Caribou Biosciences
- Tina Albertson, MD, PhD, chief medical officer, Caribou Biosciences

A live webcast of the presentation will be accessible via Caribou's website on the Events page. The archived webcast will be available on the Caribou website for 30 days after the event.

About CB-011
CB-011 is an allogeneic anti-BCMA CAR-T cell therapy being evaluated in patients with relapsed or refractory multiple myeloma (r/r MM) in the CaMMouflage phase 1 trial. To Caribou's knowledge, CB-011 is the first allogeneic CAR-T cell therapy in the clinic that is engineered to enable 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.

About the CaMMouflage phase 1 clinical trial
The CaMMouflage clinical trial is a multicenter, open-label phase 1 trial evaluating CB-011 in adults with relapsed or refractory multiple myeloma (r/r MM) who have been treated with three or more prior lines of therapy. Using a 3+3 dose escalation design, safety and efficacy of CB-011 were evaluated in 48 patients at multiple dose levels and two different lymphodepletion regimens. Thirteen patients were treated with a single dose of CB-011 ($50x10^6$ [N=3], $150x10^6$ [N=7], and $450x10^6$ [N=3] CAR-T cells) with a lymphodepletion (LD) regimen of 300 mg/m^2 cyclophosphamide and 30 mg/m^2 fludarabine daily for three days, and 35 patients were treated with a single dose of CB-011 ($150x10^6$ [N=6], $300x10^6$ [N=13], $450x10^6$ [N=13], and $800x10^6$ [N=3] CAR-T cells) with a LD regimen of 500 mg/m^2 cyclophosphamide and 30 mg/m^2 fludarabine daily for three days. The dose expansion portion of the trial will evaluate safety and efficacy of CB-011 at $450x10^6$ CAR-T cells with the selected LD of 500 mg/m^2 cyclophosphamide and 30 mg/m^2 fludarabine daily for three days. Additional information on the CaMMouflage trial (NCT05722418) can be found at clinicaltrials.gov.

About Caribou Biosciences, Inc.
Caribou 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 focused on vispacabtagene regedleucel (vispa-cel) and CB-011 as off-the-shelf CAR-T cell therapies that have the potential to provide broad access and rapid treatment for patients with hematologic malignancies. Follow the Company @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, but are not limited to, any statements regarding the initiation, timing, progress, strategy, plans, objectives, expectations (including as to the results) with respect to the Company's CAR-T cell therapy product candidate clinical trials, including its expectations and timing regarding initiating dose expansion by the end of 2025 and reporting dose expansion data, along with longer follow-up data on dose escalation, in 2026 from its ongoing CaMMouflage phase 1 clinical trial for CB-011 in patients with relapsed or refractory multiple myeloma; its ability to successfully develop its CAR-T cell product candidates and to obtain and maintain regulatory approval for these product candidates; the likelihood of its clinical trials demonstrating safety and efficacy of its CAR-T cell therapy product candidates; the beneficial characteristics, safety, efficacy, therapeutic effects, and potential advantages of its CAR-T cell therapy product candidates; and the expected timing or likelihood of regulatory filings and approval for its CAR-T cell therapy product candidates. 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 allogeneic CAR-T cell therapy products; uncertainties related to the initiation, cost, timing, progress, and results of its current and future clinical trials; the risk that initial, preliminary, or interim clinical trial data will not ultimately be predictive of the safety and efficacy of its CAR-T cell therapy product candidates or that clinical outcomes may differ as patient enrollment continues and as more patient data becomes available; the risk that different conclusions or considerations are reached once additional data have been received and fully evaluated; the ability to obtain key regulatory input and approvals; and risks related to its limited operating history, history of net operating losses, financial position, and its ability to raise additional capital as needed to fund its operations and CAR-T cell therapy product candidate development; as well as other risk factors described from time to time in Caribou's filings with the Securities and Exchange Commission (SEC), including its Annual Report on Form 10-K for the year ended December 31, 2024, and subsequent SEC 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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