Caribou Biosciences Initiates the CB-010 GALLOP Phase 1 Trial in Lupus and Provides Outlook for Multiple Clinical Datasets in 2025

January 12, 2025

-- CB-010 GALLOP Phase 1 trial initiated in lupus --

-- CB-012 AMpLify Phase 1 trial in r/r AML completes dose level 3 with no DLTs; enrolling patients at dose level 4 --

-- CB-010 ANTLER 2L LBCL and CB-011 CaMMouflage r/r MM Phase 1 clinical data expected in H1 2025 --

-- Sri Ryali appointed chief financial officer --

-- Caribou to present at 43rd Annual J.P. Morgan Healthcare Conference on Thursday, January 16, at 10:30 am PST --

BERKELEY, Calif., Jan. 12, 2025 (GLOBE NEWSWIRE) -- Caribou Biosciences, Inc. (Nasdaq: CRBU), a leading clinical-stage CRISPR genome-editing biopharmaceutical company, today announced initiation of the GALLOP Phase 1 clinical trial evaluating CB-010 in patients with lupus nephritis (LN) and extrarenal lupus (ERL). In addition, Caribou highlighted successful execution across its three clinical-stage allogeneic CAR-T cell therapy programs in hematologic malignancies over the past year and provided an outlook on multiple clinical data catalysts planned for 2025.

"We are excited to share we have initiated the GALLOP Phase 1 trial. This milestone is a testament to the significant momentum we achieved across all four clinical programs throughout 2024," said Rachel Haurwitz, PhD, Caribou's president and chief executive officer. "In the first half of 2025, we plan to report clinical data from our lead program, CB-010, in second-line and CD19 relapsed large B cell lymphoma patients as well as dose escalation data from our second program, CB-011, in relapsed or refractory multiple myeloma. Pending confirmation of our HLA matching strategy, we plan to initiate a pivotal Phase 3 trial for CB-010 in second-line large B cell lymphoma in the second half of 2025. We are excited to be on the forefront of a new era for allogeneic CAR-T cell therapies, which offer broad access and rapid availability to patients and healthcare systems."

2024 clinical highlights and corporate accomplishments

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

- At the 2024 American Society of Clinical Oncology (ASCO) Annual Meeting, Caribou presented clinical data from the ongoing ANTLER Phase 1 clinical trial that indicated a single dose of CB-010 has the potential to be on par with the safety, efficacy, and durability of approved autologous CAR-T cell therapies.
- A retrospective analysis of all patient data demonstrated that patients who received a dose of CB-010 manufactured from a donor with ≥4 matching human leukocyte antigen (HLA) alleles showed improved progression free survival (PFS).
- To confirm the HLA matching strategy, Caribou is enrolling approximately 20 additional second-line large B cell lymphoma (2L LBCL) patients in the ongoing ANTLER Phase 1 clinical trial.
- Caribou also began enrolling a proof-of-concept cohort of up to 10 patients who have relapsed following any prior CD19-targeted therapy in this population of unmet need.
- Caribou plans to present data from both the additional 2L and prior CD19 relapsed LBCL patient cohorts in H1 2025. The company 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 results in outcomes on par with autologous CAR-T cell therapies. 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

- Caribou expanded the CB-010 program following clearance of its Investigational New Drug (IND) application by the U.S. Food and Drug Administration (FDA) for CB-010 in LN and ERL.
- The FDA granted Fast Track designation to CB-010 for refractory systemic lupus erythematosus (SLE).
- Caribou has initiated the GALLOP Phase 1 clinical trial, an open-label, multicenter clinical trial designed to evaluate a single infusion of CB-010 in adult patients with LN and ERL.

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 implemented a lymphodepletion regimen that includes a deeper 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). Following observations of efficacy, Caribou is enrolling additional patients as backfill at active dose levels with the deeper lymphodepletion regimen.
- 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.

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

- The 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 3 ($150x10^6$ CAR-T cells, N=3) and no DLTs were observed. Patients are being enrolled at dose level 4 ($300x10^6$ CAR-T cells).

Corporate updates
Appointed experienced executive leaders and expanded the scientific advisory board (SAB)

- In January 2024, Tim Kelly was appointed chief technology officer and he leads Caribou's process development and manufacturing organizations.
- In August 2024, Tina Albertson, MD, PhD, was appointed chief medical officer and she leads Caribou's clinical programs and the clinical, regulatory, and medical affairs functions.
- In January 2025, Sri Ryali was appointed chief financial officer and he leads Caribou's corporate finance, investor relations, and corporate communications functions.
- Terri Laufer, MD, was appointed to Caribou's SAB. She is a leading rheumatologist known for her extensive research into immune cell regulation and dysfunction that leads to autoimmune diseases.

$281M in cash, cash runway into H2 2026

- Caribou previously reported $281 million in cash, cash equivalents, and marketable securities as of September 30, 2024, which is expected to fund the current operating plan into H2 2026.

2025 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 drives outcomes that are on par with autologous CAR-T cell therapies.
- **CB-010 GALLOP:** Caribou plans to provide updates as the GALLOP Phase 1 clinical trial in LN and ERL advances.
- **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.

Caribou to present at the 43rd Annual J.P. Morgan Healthcare Conference
Dr. Haurwitz is scheduled to present a corporate update at the 43rd Annual J.P. Morgan Healthcare Conference on Thursday, January 16, 2025 at 10:30 am PST.

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-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 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) and GALLOP trial (NCT06752876) 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 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 to offer broad access and rapid availability of 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 regarding plans to present or provide (i) ANTLER Phase 1 clinical trial data in H1 2025 from both the additional 2L and prior CD19 relapsed LBCL patient cohorts and timing and commencement of an ANTLER pivotal Phase 3 clinical trial; (ii) dose escalation data in H1 2025 from the ongoing CaMMouflage Phase 1 clinical trial for CB-011 in r/r MM; (iii) updates from dose escalation from the AMpLify Phase 1 clinical trial for CB-012; and (iv) updates from the GALLOP Phase 1 clinical trial for CB-010 in patients with LN and ERL; as well as 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 its current and future research and development programs, preclinical studies, and clinical trials; 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; 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 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, 2023 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.

Caribou Biosciences, Inc. contacts:
Investors:
Amy Figueroa, CFA
investor.relations@cariboubio.com

Media:
Peggy Vorwald, PhD
media@cariboubio.com