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Cabaletta Bio Presents Updated Interim DesCAARTes™ Trial Phase 1 Data at the ASGCT 25th Annual Meeting

PHILADELPHIA, May 18, 2022 (GLOBE NEWSWIRE) -- Cabaletta Bio, Inc. (Nasdaq: CABA), a clinical-stage biotechnology company focused on the discovery and development of targeted cell therapies for patients with autoimmune diseases, today presented updated clinical and translational data through 6 months of follow-up in cohorts A1 through A3, safety data through 3 months and persistence data through 1 month of follow-up in cohorts A1 through A4 from the DesCAARTes™ trial at the American Society of Gene & Cell Therapy (ASGCT) 25th Annual Meeting being held in Washington, D.C. from May 16-19, 2022.

“At ASGCT, we presented updated interim data showing that DSG3-CAART has had a favorable safety profile with no dose limiting toxicities or cytokine release syndrome of any grade through cohort A4, which was a dose of 2.5 billion DSG3-CAART cells. In addition, we have observed a dose dependent increase in DSG3-CAART persistence, which at cohort A4 approached the lower end of the range that is observed in anti-CD19 CART oncology studies in combination with lymphodepletion,” said David Chang, M.D., Chief Medical Officer of Cabaletta. “These data continue to support the planned dose escalation in this trial and our conviction in the potential of this program. As we progress through additional cohorts of the study, we look forward to evaluating the relationship between DSG3-CAART persistence and potential clinical responses in patients with mPV along with the opportunity to explore higher doses and an enhanced manufacturing process to meet our goal of reaching deep, durable and potentially curative responses for these patients.”

Cabaletta’s DesCAARTes™ Phase 1 trial is an open-label, dose escalation, multi-center study of DSG3-CAART in adults with mucosal-dominant pemphigus vulgaris (mPV). The trial is designed to determine the maximum tolerated dose of DSG3-CAART in adult subjects with active, anti-DSG3 Ab positive, biopsy confirmed mPV that is inadequately managed by one or more standard therapies. The primary endpoint is incidence of adverse events (AEs), including dose-limiting toxicities (DLTs), such as cytokine release syndrome (CRS) and neurotoxicity, related to DSG3-CAART within three months of infusion. Secondary endpoints include CAART persistence (qPCR), anti-DSG3 Ab levels (ELISA) and disease activity (PDAI). The trial is currently in cohort A5 (5.0 to 7.5 billion cells) and is being conducted across multiple clinical sites throughout the United States. The Company plans to include two new additional dose cohorts – A5e (enhanced manufacturing process at 5.0 to 7.5 billion cells) and A6m (multi-dose regimen at 10 to 15 billion cells). The prioritization of cohorts following cohort A5 (e.g. A5e or A6m) is subject to evaluation of emerging data and discussions with the FDA, as applicable.

The updated interim DesCAARTes™ trial Phase 1 data included 12 treated subjects, four cohorts with three patients per cohort; nine having completed six months follow up after DSG3-CAART infusion. The posters as presented at ASGCT are available at https://www.cabalettabio.com/technology/posters-publications. The data show:

- No DLTs, CRS of any grade, or related serious AEs were observed in any subject within three months of DSG3-CAART infusion through cohort A4 (2.5 billion cells).
- There was a dose dependent increase in DSG3-CAART persistence through day 29 in cohorts A1 to A4, indicating that DSG3-CAART cells were not eliminated through immune-mediated rejection.
    - Persistence (AUC_{d29} and C_{max}) in cohort A4 approached the lower end of the range that is observed in clinical trials of anti-CD19 CART combined with lymphodepletion in B-cell malignancies
- In cohorts A1 to A3:
    - Disease activity was clear or almost clear (PDAI 0-1) in 0/9 subjects at screening, 1/9 at pre-infusion, 2/9 at month one, 5/9 at month two, 3/9 at month three, 2/9 at month four, 3/9 at month five and 1/9 at month six after treatment.
    - Through six months post DSG3-CAART infusion, no clear pattern was observed in changes in anti-DSG3 Ab levels (ELISA) or disease activity (PDAI) in the low dose cohorts where the A3 dose (500 million cells) represents 6.7 to 10% of the ongoing cohort A5 dose (5.0 to 7.5 billion cells).
- One subject from cohort A1 was retreated with 500 million cells (the cohort A3 dose) and persistence in the subject was similar to the three subjects who were originally administered the cohort A3 dose, suggesting that there was not immune-mediated clearance of DSG3-CAART cells after retreatment and repeat dosing of patients is possible, if indicated.

Data presented on the company's manufacturing process were as follows:

- In cohorts A1-A4, the manufacturing success rate was 100% with functional DSG3-CAART cells manufactured successfully from mPV patient apheresis material.
- Infused DSG3-CAART cells exhibited a stem cell or central memory phenotype with a strong positive correlation between the dose of gene modified T cells and post-infusion persistence to day 29.
- These data suggest that DSG3-CAART cells are not being eliminated by the pre-existing anti-DSG3 immunity present in mPV.

About Cabaletta Bio
Cabaletta Bio (Nasdaq: CABA) is a clinical-stage biotechnology company focused on the discovery and development of engineered T cell therapies that have the potential to provide a deep and durable, perhaps curative, treatment for patients with autoimmune diseases. The CABA™ platform, in combination with Cabaletta Bio's proprietary technology, has advanced a growing pipeline that currently includes potential treatments for patients with mucosal pemphigus vulgaris, MuSK-associated myasthenia gravis, PLA2R-associated membranous nephropathy, mucocutaneous pemphigus vulgaris and hemophilia A with FVIII alloantibodies. Cabaletta Bio's headquarters are located in Philadelphia, PA. For more information, visit www.cabalettabio.com and follow us on LinkedIn.

Forward-Looking Statements
This press release contains "forward-looking statements" of Cabaletta Bio within the meaning of the Private Securities Litigation Reform Act of 1995, as amended, including without limitation, express or implied statements regarding expectations regarding: Cabaletta's ability to grow its autoimmune-focused pipeline; the progress and results of its DesCAARTes™ Phase 1 trial, including Cabaletta's ability to enroll the requisite number of

patients, dose each dosing cohort in the intended manner, and progress the trial; the expected significance and impact around the clinical and translational data updates from cohorts A1 through A3 of the DesCAARTes™ trial, including 3-month safety and 1-month persistence data in cohorts A1 through A4, described herein; the expected timing and significance of the announcement of 28-day safety for cohort A5 and clinical and translational data for cohort A4 in mid-2022; the expectation that Cabaletta may improve outcomes for patients suffering from mPV; Cabaletta's ability to continue progressing in cohort A5, including the planned addition of an enhanced manufacturing process; Cabaletta's ability to escalate dosing as high as 10 to 15 billion cells in a planned future cohort or otherwise; Cabaletta's ability to advance dose escalation in the DesCAARTes™ Phase 1 trial at the current dose ranges for the current cohorts and any projected potential dose ranges for future cohorts, and to optimize its targeted cell therapy; Cabaletta's ability to evaluate, and the potential significance of, the relationship between DSG3-CAART persistence and potential clinical responses in patients with mPV; Cabaletta's ability to safely retreat additional patients and whether Cabaletta will continue to observe a lack of immune-mediated clearance of DSG3-CAART cells after retreatment and repeat dosing of patients; the expectation that Cabaletta Bio may improve outcomes for patients suffering from MuSK MG; plans to initiate patient dosing in an open-label Phase 1 clinical trial to evaluate MuSK-CAART safety and tolerability in MuSK MG patients in 2022; Cabaletta's plans to advance development of its preclinical pipeline; presentation of additional data at upcoming scientific conferences, and other preclinical data; expectations regarding the design, implementation, timing and success of its current and planned clinical trials and the successful completion of nonclinical studies; planned potential timing and advancement of its preclinical studies and clinical trials and related regulatory submissions; ability to optimize the impact of its collaborations on its development programs; the impact of COVID-19 on the timing, progress, interpretability of data, and results of ongoing or planned preclinical and clinical trials; statements regarding the timing of regulatory filings regarding its development programs.

Any forward-looking statements in this press release are based on management's current expectations and beliefs of future events, and are subject to a number of risks and uncertainties that could cause actual results to differ materially and adversely from those set forth in or implied by such forward-looking statements. These risks and uncertainties include, but are not limited to: the risk that signs of biologic activity or persistence may not inform long-term results; Cabaletta's ability to demonstrate sufficient evidence of safety, efficacy and tolerability in its preclinical and clinical trials of DSG3-CAART; the risk that persistence observed with effective CART-19 oncology studies in combination with lymphodepletion is not indicative of, or applicable to, clinical responses in patients with mPV; risks related to clinical trial site activation or enrollment rates that are lower than expected; risks related to unexpected safety or efficacy data observed during clinical studies; risks related to the impact of public health epidemics, such as the ongoing COVID-19 pandemic, affecting countries or regions in which we have operations or do business; Cabaletta's ability to retain and recognize the intended incentives conferred by Orphan Drug Designation and Fast Track Designation for DSG3-CAART for improving healing of mucosal blisters in patients with mucosal pemphigus vulgaris; Cabaletta's ability to demonstrate sufficient evidence of safety, efficacy and tolerability in its preclinical and clinical trials of DSG3-CAART; risks related to Cabaletta's ability to protect and maintain its intellectual property position; uncertainties related to the initiation and conduct of studies and other development requirements for its product candidates; the risk that any one or more of Cabaletta's product

candidates will not be successfully developed and commercialized; and the risk that the initial or interim results of preclinical studies or clinical studies will not be predictive of future results in connection with future studies. For a discussion of these and other risks and uncertainties, and other important factors, any of which could cause Cabaletta's actual results to differ from those contained in the forward-looking statements, see the section entitled "Risk Factors" in Cabaletta's most recent annual report on Form 10-K as well as discussions of potential risks, uncertainties, and other important factors in Cabaletta's other filings with the Securities and Exchange Commission. All information in this press release is as of the date of the release, and Cabaletta undertakes no duty to update this information unless required by law.

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