

May 3, 2021

Cabaletta Bio®

# Cabaletta Bio Reports Acute Safety Data from the First Dose Cohort in DesCAARTes™ Trial

- *No dose limiting toxicities (DLTs) or clinically relevant adverse events observed in the first dose cohort as of April 30, 2021 –*
- *Second dose cohort to be initiated after the third patient completes 28 day follow up, absent any DLT –*
- *Acute safety data from the second and third cohorts are anticipated in the third and fourth quarters of 2021, respectively. Topline data on target engagement in the first cohort are expected in the second half of 2021 –*
- *Company to host conference call today at 8:30 a.m. ET–*

PHILADELPHIA, May 03, 2021 (GLOBE NEWSWIRE) -- Cabaletta Bio, Inc. (Nasdaq: CABA), a clinical-stage biotechnology company focused on the discovery and development of engineered T cell therapies for patients with B cell-mediated autoimmune diseases, today announced acute safety data from the first dose cohort of the ongoing DesCAARTes™ Phase 1 clinical trial of DSG3-CAART for the treatment of patients with mucosal-dominant pemphigus vulgaris (mPV).

“We are encouraged by the acute safety profile of DSG3-CAART in this initial low dose cohort. In the first cohort of three patients dosed with DSG3-CAART, there were no clinically relevant adverse events, including cytokine release syndrome or neurotoxicity, during the 8-day acute safety window, which we expect is the period with highest probability of observing treatment-related toxicities. In addition, no dose-limiting toxicities or clinically relevant adverse events were observed in the two patients who have completed more than the full 28-day DLT monitoring period post-infusion,” said David J. Chang, M.D., Chief Medical Officer of Cabaletta. These safety data were observed with an administered dose of 20 million DSG3-CAART cells, without preconditioning and in the presence of circulating anti-DSG3 antibodies; DSG3-CAART was detected at low levels via quantitative polymerase chain reaction in both patients who have been evaluated and completed the 28-day DLT period. The third patient is scheduled to be evaluated for presence of DSG3-CAART after the 28-day follow-up period.

“The pace of the clinical trial is accelerating with the ongoing enrollment of patients and engagement of additional clinical sites. We believe these initial safety data represent an important step towards achieving our goal to offer a therapy that may provide deep and durable responses, and potentially cures, for patients in the pemphigus community,” said Dr. Chang.

The DesCAARTes™ trial is currently enrolling patients in the second cohort at a treatment dose of 100 million DSG3-CAART cells. Infusions are planned to initiate following the third

patient in the first dose cohort completing the 28-day monitoring period without any DLTs. Cabaletta expects to announce acute safety data for the second and third cohorts in the third and fourth quarters of 2021, respectively. Topline data on target engagement from the first cohort are anticipated during the second half of 2021. Cabaletta will continue to provide additional topline safety and target engagement data from the DesCAARTes™ trial once available on a cohort-by-cohort basis.

### **Conference Call Details**

Cabaletta management will host a conference call today at 8:30 a.m. ET to discuss this data and other recent pipeline updates. To participate in the conference call, please dial 866-939-3921 (domestic) or 678-302-3550 (international) and refer to the conference ID 50150570. A live webcast of the presentation can be accessed under “Events & Presentations” in the Investors & Media section of Cabaletta’s website at [www.cabalettabio.com](http://www.cabalettabio.com).

### **About the DesCAARTes™ Clinical Trial**

Cabaletta’s DesCAARTes™ Phase 1 trial is an open-label, multi-center study of DSG3-CAART in adults with mucosal-dominant pemphigus vulgaris. The trial is designed to evaluate the safety and tolerability of DSG3-CAART as well as to identify evidence of target engagement and early signs of efficacy. The study consists of three parts: 1) dose escalation, 2) dose consolidation, and 3) cohort expansion at the final selected dose and schedule. The trial is expected to enroll approximately 30 patients across multiple clinical sites throughout the United States. Visit [clinicaltrials.gov](http://clinicaltrials.gov) ([NCT04422912](https://clinicaltrials.gov/ct2/show/study/NCT04422912)) for more information.

### **About Pemphigus Vulgaris**

mPV is a rare autoimmune blistering disease that is characterized by the loss of adhesion between cells of the skin or mucous membranes. mPV is caused by the production of autoantibodies that disrupt structural proteins within the skin and/or mucosa that connect with other proteins to enable the skin and/or mucosal cells to connect with each other. The autoantibodies can target DSG3 and/or desmoglein 1 (DSG1), which are primarily expressed in the mucosal membranes and skin, respectively. mPV is characterized by autoantibodies against DSG3 only whereas mucocutaneous PV (mcPV) is characterized by autoantibodies against DSG3 and DSG1.

### **About CAAR T Cell Therapy**

Chimeric AutoAntibody Receptor (CAAR) T cells are designed to selectively bind and eliminate only disease-causing B cells, while sparing the normal B cells that are essential for human health. CAAR T cells are based on the chimeric antigen receptor (CAR) T cell technology. While CAR T cells typically contain a CD19-targeting molecule, CAAR T cells express an autoantibody-targeted antigen on their surface. The co-stimulatory domain and the signaling domain of both a CAR T cell and a CAAR T cell carry out the same activation and cytotoxic functions. Thus, Cabaletta’s CAARs are designed to direct the patient’s T cells to kill only the pathogenic cells that express disease-causing autoantibodies on their surface, potentially leading to complete and durable remission of disease while sparing all other B cell populations that provide beneficial immunity from infection.

### **About Cabaletta Bio**

Cabaletta Bio is a clinical-stage biotechnology company focused on the discovery and development of engineered T cell therapies, and exploring their potential to provide a deep

and durable, perhaps curative, treatment, for patients with B cell-mediated autoimmune diseases. The Cabaletta Approach to selective B cell Ablation (CABA™) platform, in combination with Cabaletta's proprietary technology, utilizes CAAR T cells that are designed to selectively bind and eliminate only specific autoantibody-producing B cells while sparing normal antibody-producing B cells, which are essential for human health. The Company's lead product candidate, DSG3-CAART, is being evaluated in the DesCAARTes™ phase 1 clinical trial as a potential treatment for patients with mucosal pemphigus vulgaris, a prototypical B cell-mediated autoimmune disease. The FDA granted Fast Track Designation for DSG3-CAART in May 2020. For more information about the DesCAARTes™ Phase 1 clinical trial, please see [www.clinicaltrials.gov](http://www.clinicaltrials.gov). The Company's lead preclinical product candidate, MuSK-CAART, is in IND-enabling studies and is designed as a potential treatment for patients with MuSK-associated myasthenia gravis. For more information, visit [www.cabalettabio.com](http://www.cabalettabio.com).

### **Forward-Looking Statements**

This press release contains "forward-looking statements" of Cabaletta within the meaning of the Private Securities Litigation Reform Act of 1995, as amended, including without limitation, express or implied statements regarding expectations regarding the progress and results of its DesCAARTes™ Phase 1 trial, including Cabaletta's ability to enroll the requisite number of patients in the trial; the expectation that Cabaletta may improve outcomes for patients suffering from mPV; the effectiveness and timing of product candidates that Cabaletta may develop, including in collaboration with academic partners; the safety, efficacy and tolerability of DSG3-CAART for the treatment of mPV; expectations of the potential impact of COVID-19 on strategy, future operations, and the timing of its clinical trials, including the potential impacts on enrollment and initiation of its DesCAARTes™ Phase 1 trial; and statements regarding 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: Cabaletta's ability to demonstrate sufficient evidence of safety, efficacy and tolerability in its clinical trials of DSG3-CAART; 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 affecting countries or regions in which we have operations or do business, such as COVID-19; 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; and the risk that the initial or interim results of 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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