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Cabaletta Bio Announces First Patient Dosed in Landmark DesCAARTes™ Trial of DSG3-CAART for Treatment of Mucosal-Dominant Pemphigus Vulgaris

First patient ever infused with a CAAR (chimeric autoantibody receptor) T cell therapy product candidate expands the potential clinical application of CAR T technology beyond cancer into autoimmune diseases

PHILADELPHIA, Dec. 08, 2020 (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 that the first patient has been dosed in the DesCAARTes™ Phase 1 clinical trial of DSG3-CAART for the treatment of patients with mucosal-dominant pemphigus vulgaris (mPV).

“This is an important milestone in the development of our lead product candidate, DSG3-CAART, for patients with mucosal pemphigus vulgaris and for patients with B cell mediated autoimmune diseases more generally. We believe this is the first time a highly targeted, antigen specific cell therapy has been dosed in a patient with autoimmune disease. The study is designed to provide insights into the clinical effect of our precision CAAR T cell therapy in patients suffering from mPV,” said David J. Chang, M.D., Chief Medical Officer of Cabaletta Bio. “Currently available therapies for mPV patients, including steroids, typically induce broad immunosuppression, offer modest efficacy and/or are associated with frequent relapses. DSG3-CAART therapy, which is engineered to target and specifically eliminate the cells responsible for generating disease-causing autoantibodies while preserving the healthy immune system, provides mPV patients the potential of a deep and durable response, perhaps even a cure.”

“This is a huge accomplishment that will advance the entire field of cell therapy,” stated Carl H. June, M.D., who is a member of the Cabaletta Bio Scientific Advisory Board and the Richard W. Vague Professor of Immunotherapy in the Department of Pathology and Laboratory Medicine at the Perelman School of Medicine at the University of Pennsylvania, “The precise CAAR T technology which was discovered at Penn and is being developed by Cabaletta Bio builds on the legacy of commercially-approved CAR T therapies, to offer the promise of deep and durable responses beyond oncology to patients with autoimmune diseases.”

About the DesCAARTes™ Clinical Trial
Cabaletta Bio’s DesCAARTes™ Phase 1 trial is an open-label, multi-center study of DSG3-CAART in adults with mucosal-dominant pemphigus vulgaris (mPV). 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) expansion at the final selected dose and schedule. The trial is expected to enroll approximately 30 subjects across multiple clinical sites throughout the United States. Visit clinicaltrials.gov (NCT04422912) for more information.

About Pemphigus Vulgaris
PV is a rare autoimmune blistering disease that is characterized by the loss of adhesion between cells of the skin or mucous membranes. PV 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 Bio'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 Chimeric AutoAntibody Receptor (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 clinical trial, please see 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.

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 the progress and results of its DesCAARTes™ Phase 1 trial, including Cabaletta Bio's ability to enroll the requisite number of patients and dosing of its first patient; the expectation that

Cabaletta Bio 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; the impact of preclinical data on the future development of CAAR T therapies in our pipeline portfolio expectations of the potential impact of COVID-19 on strategy, future operations, and the timing of its clinical trials, including the potential impacts on 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 Bio's ability to demonstrate sufficient evidence of safety, efficacy and tolerability in its preclinical and 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; Cabaletta's ability to retain and recognize the intended incentives conferred by Orphan Drug Designation for DSG3-CAART for the treatment of PV and Fast Track Designation for DSG3-CAART for the treatment of mPV; 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 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.

Editor's Note: Dr. June is a University of Pennsylvania faculty member and holds an equity stake in the Company.

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