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Cabaletta Bio Receives IND Clearance from FDA to Initiate First Clinical Trial of DSG3-CAART in Patients with Mucosal Pemphigus Vulgaris

First IND clearance of a product candidate derived from the CABA™ platform

PHILADELPHIA, Oct. 01, 2019 (GLOBE NEWSWIRE) -- Cabaletta Bio, Inc. (Company), a clinical-stage biotechnology company focused on the discovery and development of engineered T cell therapies for the treatment of patients with B cell-mediated autoimmune diseases, today announced that it has received clearance of its Investigational New Drug (IND) application from the U.S. Food and Drug Administration (FDA) to initiate a first-in-human clinical trial of desmoglein 3 chimeric autoantibody receptor T cells (DSG3-CAART) in patients with mucosal pemphigus vulgaris (mPV) to assess the safety and tolerability of DSG3-CAART in these patients. The Company anticipates enrolling the first patient in 2020.

“The FDA’s clearance of our IND for DSG3-CAART is an important milestone for patients with mPV and the first IND clearance for a product candidate from our Cabaletta Approach to selective B cell Ablation (CABA™) platform,” said Steven Nichtberger, M.D., Chief Executive Officer and Co-Founder of Cabaletta Bio. “DSG3-CAART is the first of several CAAR T cell product candidates in our announced pipeline, which includes product candidates targeting patients with MuSK myasthenia gravis, the mucocutaneous form of pemphigus vulgaris (PV), and hemophilia A patients with inhibitors to factor VIII therapy.”

mPV is a potentially fatal, B cell-mediated chronic, rare autoimmune disease that causes painful blisters and sores on mucous membranes of affected patients, leading to severe and sometimes debilitating and life-altering effects. DSG3-CAART is designed to selectively target and eliminate B cells expressing autoantibodies specific for DSG3 that are the cause of mPV while preserving healthy B cell immune function. DSG3-CAART has the potential to generate persistent complete remission off therapy while avoiding the adverse effects of chronic and generalized immunosuppression. Currently available treatment options induce broad immunosuppression, which put the patient at risk of infection and often provide only transient complete remission with subsequent relapses for patients with moderate to severe mPV. Approximately 4,250 patients suffer from mPV in the United States and 6,250 patients in Europe, which accounts for approximately 25% of all PV cases.

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 desmoglein 3 (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 developed at the University of Pennsylvania. 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 for 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. Cabaletta's lead product candidate is based on the Chimeric Antigen Receptor (CAR) T cell technology developed at the University of Pennsylvania (Penn) that resulted in one of the first commercially-available CAR T cell products for the treatment of B cell cancers. Cabaletta was founded by Penn physician/scientists Michael Milone, M.D., Ph.D., and Aimee Payne, M.D., Ph.D., who serve as co-chairs of Cabaletta's Scientific Advisory Board, and Steven Nichtberger, M.D., CEO of Cabaletta. Cabaletta has an exclusive global licensing agreement and multiple sponsored research agreements with the University of Pennsylvania to develop the CAAR T technology to treat B cell-mediated autoimmune diseases. The Company's lead product candidate is being studied as a potential treatment for a prototypical B cell-mediated autoimmune disease, mucosal pemphigus vulgaris (mPV). For more information, visit www.cabalettabio.com.

Cautionary Note Regarding Forward-Looking Statements

This press release contains forward-looking statements within the meaning of the Private Securities Litigation Reform Act of 1995, as amended, including, without limitation, those regarding the planned timing, enrollment and results for our Phase 1 clinical trial for DSG3-CAART for the treatment of mPV, our expectations regarding the results from our Phase 1 clinical trial for DSG3-CAART and the FDA's review of the results therefrom, our expectations regarding the ability of DSG3-CAART to treat mPV, the planned timing for additional IND applications, the expected timing and progress of preclinical studies and clinical trials for our other product candidates based on our CABA platform, our ability to meet the objectives of our planned preclinical studies and clinical trials and demonstrate the safety and efficacy of our product candidates, our ability to fund the development of our

CABA platform product candidates, the development of our CABA platform product candidates and their therapeutic potential, whether and when, if at all, our CABA platform product candidates will receive approval from the FDA and for which, if any, indications, and competition from other biotechnology companies. The words “may,” “will,” “could,” “would,” “should,” “expect,” “plan,” “anticipate,” “intend,” “believe,” “estimate,” “predict,” “project,” “potential,” “continue,” “target” and similar expressions are intended to identify forward-looking statements, although not all forward-looking statements contain these identifying words. Any forward-looking statements in this press release are based on management’s current expectations and beliefs and are subject to a number of risks, uncertainties and important factors that may cause actual events or results to differ materially from those expressed or implied by any forward-looking statements contained in this press release, including, without limitation, risks and uncertainties related to the delay of any current or planned preclinical studies or clinical trials or the development of our product candidates, including DSG3-CAART, our ability to successfully demonstrate the efficacy and safety of our drug candidates including in later-stage studies and trials, the preclinical and clinical results for our product candidates, which may not support further development of such product candidates, actions of regulatory agencies, any or all of which may affect the initiation, timing and progress of preclinical studies, clinical trials and regulatory development. We caution you not to place undue reliance on any forward-looking statements, which speak only as of the date they are made. We disclaim any obligation to publicly update or revise any such statements to reflect any change in expectations or in events, conditions or circumstances on which any such statements may be based, or that may affect the likelihood that actual results will differ from those set forth in the forward-looking statements. Any forward-looking statements contained in this press release represent our views only as of the date hereof and should not be relied upon as representing its views as of any subsequent date. We explicitly disclaim any obligation to update any forward-looking statements.

Editor’s Note: Drs. Payne, Milone and Nichtberger are University of Pennsylvania faculty members and hold equity stakes in the Company, and the University of Pennsylvania is an equity holder and investor in the Company. In addition, both Penn and the inventors of the licensed technology may receive additional financial benefits under the license in the future.

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Cabaletta Bio™

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