

FDA Grants DSG3-CAART Orphan Drug Designation for the Treatment of Pemphigus Vulgaris

PHILADELPHIA, Jan. 29, 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 U.S. Food and Drug Administration (FDA) has granted Orphan Drug Designation for the Company's lead product candidate, DSG3-CAART, for the treatment of pemphigus vulgaris (PV). DSG3-CAART is designed to target the cause of mucosal PV (mPV), B cells that express pathogenic autoantibodies directed against the DSG3 protein, while preserving normal B cell immune function.

"Mucosal pemphigus vulgaris is a rare and potentially fatal, chronic autoimmune disease characterized by the loss of adhesion between cells of mucous membranes, resulting in widespread damage, painful blisters of the mucosal membranes, and increased susceptibility to life-threatening systemic infections," said David Chang, M.D., Chief Medical Officer of Cabaletta. "For affected patients, despite current treatment options, there is an urgent unmet need for more effective and durable therapies that can provide reliable, complete, and persistent remission from the disease beyond general immune suppression and B cell depletion provided by current treatment options. Orphan Drug Designation is an important recognition for investigational therapies for rare diseases and provides us with potentially valuable benefits as we prepare to initiate the DesCAARTes trial to generate and then report acute safety data from the first cohort of patients by the end of 2020."

The FDA grants Orphan Drug Designation to drugs or biologics intended to treat or prevent rare diseases or conditions that affect fewer than 200,000 individuals in the United States. This designation qualifies Cabaletta for certain incentives, which may include partial tax credit for clinical trial expenditures, waived user fees and potential eligibility for seven years of marketing exclusivity.

About Pemphigus Vulgaris

PV is a rare autoimmune blistering disease that is characterized by painful blisters and an increased risk of life-threatening infections due to 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 by scientists 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 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. Cabaletta's lead product candidate is based on the Chimeric Antigen Receptor (CAR) T cell technology developed by scientists at the University of Pennsylvania that resulted in the first commercially-available CAR T cell products for the treatment of B cell cancers. 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 in a phase 1 clinical trial as a potential treatment for a prototypical B cell-mediated autoimmune disease, mucosal pemphigus vulgaris. 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 PV, 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 PV, our expectations regarding the intended incentives conferred by Orphan Drug Designation for DSG3-CAART for the treatment of PV, the expected timing, progress and success 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 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 retain and recognize the intended incentives conferred by Orphan Drug Designation for DSG3-CAART for the treatment of PV, 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.

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Source: Cabaletta Bio