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Cabaletta Bio Receives FDA Fast Track Designation for CABA-201

PHILADELPHIA, May 01, 2023 (GLOBE NEWSWIRE) -- Cabaletta Bio, Inc. (Nasdaq: CABA), a clinical-stage biotechnology company focused on developing and launching the first curative targeted cell therapies for patients with autoimmune diseases, today announced that the U.S. Food and Drug Administration (FDA) has granted Fast Track Designation for CABA-201, a 4-1BB-containing fully human CD19-CAR T cell investigational therapy, designed to deplete CD19-positive B cells and improve disease activity in patients with systemic lupus erythematosus (SLE) and lupus nephritis (LN). Cabaletta has been cleared to initiate a Phase 1/2 clinical trial of CABA-201 for the treatment of 6 SLE patients with active LN, and in a separate parallel cohort, 6 patients with active SLE without renal involvement, with an initial dose that is equivalent to the dose used in the September 2022 *Nature Medicine* publication of a 4-1BB containing CD19-CAR T construct evaluated in patients with SLE.

“Despite advances over the last few decades, treatment options for SLE remain inadequate. There are currently no curative options available that achieve durable disease remission. Existing therapies typically result in general immunosuppression, require chronic administration, and are often administered in conjunction with steroids and other immunosuppressive medications to reduce disease burden, which can leave patients with continued disease activity, treatment-associated side effects, and impaired quality of life,” said David J. Chang, M.D., Chief Medical Officer of Cabaletta. “We believe the FDA’s decision to grant Fast Track Designation for CABA-201 underscores the unmet need for a treatment that has the potential to provide deep and durable responses for people living with lupus and potentially other autoimmune diseases where B cells contribute to disease. We look forward to initiating the Phase 1/2 trial for CABA-201 and further evaluating its therapeutic potential for patients in need.”

CABA-201 is designed to be given as a one-time infusion, to evaluate its potential to transiently, but fully, eliminate B cells, enabling an “immune system reset” with durable remission in patients with SLE. The Phase 1/2 clinical trial is an open-label study designed to evaluate CABA-201 in SLE subjects with active LN or active SLE without renal involvement. CABA-201 will be administered at a dose of 1.0×10^6 cells/kg, and the study will enroll 6 subjects in an active LN cohort and 6 subjects in an active SLE without renal involvement cohort, in parallel. Subjects will be treated with a standard preconditioning regimen consisting of fludarabine and cyclophosphamide prior to CABA-201 infusion. This represents the first trial assessing Cabaletta’s CARTA (Chimeric Antigen Receptor T cells for Autoimmunity) strategy.

About Fast Track Designation
The FDA’s Fast Track process is intended to facilitate the expedited development and review of therapeutics intended to treat serious or life-threatening conditions and to address unmet medical needs. Companies that receive Fast Track Designation are eligible for several potential benefits, including the opportunity for more frequent meetings and

interactions with the FDA during clinical development as well as eligibility for accelerated approval and/or priority review, if relevant criteria are met. Companies may also be allowed to submit sections of their Biologics License Application on a rolling basis.

About Systemic Lupus Erythematosus
SLE is a chronic, potentially severe, autoimmune disease in which the immune system attacks healthy tissue throughout the body, most commonly impacting young women between the ages of 15 and 40 with higher frequency and greater severity in people of color. It is characterized by abnormal B cell function and autoantibody production resulting in a range of clinical manifestations including end organ damage and an increased risk of death. SLE affects an estimated 160,000-320,000 patients in the U.S, with LN as the most common end-organ manifestation, affecting approximately 40% of SLE patients. Among these patients, the risk of end-stage renal disease is approximately 17% and the risk of death is approximately 12%, each within 10 years of diagnosis.

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 encompasses two strategies: the CARTA (Chimeric Antigen Receptor T cells for Autoimmunity) strategy, with CABA-201, a 4-1BB-containing fully human CD19-CAR T, as the lead product candidate being evaluated in lupus nephritis and systemic lupus erythematosus without renal involvement, and the CAART (Chimeric AutoAntibody Receptor T cells) strategy, with multiple clinical-stage candidates, including DSG3-CAART for mucosal pemphigus vulgaris and MuSK-CAART for MuSK myasthenia gravis. The expanding CABA™ platform is designed to develop potentially curative therapies for patients with a broad range of autoimmune diseases. Cabaletta Bio's headquarters and labs are located in Philadelphia, PA.

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: Cabaletta's ability to retain and recognize the intended incentives conferred Fast Track Designation for CABA-201 to deplete CD19-positive B cells and improve disease activity in patients with SLE and LN; Cabaletta's ability to grow its autoimmune-focused pipeline; the Company's business plans and objectives; Cabaletta Bio's expectations around the potential success and therapeutic benefits of CABA-201, including its belief that CABA-201 may enable an "immune system reset" and provide deep and durable responses for patients with SLE and potentially for patients diagnosed with other autoimmune disease; the Company's plans to initiate a Phase 1/2 clinical trial of CABA-201 in patients with SLE, including its anticipated progress, clinical trial design, ability to leverage its experience in autoimmune cell therapy and lupus product development; Cabaletta's ability to enroll the requisite number of patients, dose each dosing cohort in the intended manner and advance the trial as planned in its Phase 1/2 clinical trial of CABA-201; and the ability to accelerate Cabaletta's pipeline and develop meaningful therapies for patients, including in collaboration with academic and industry partners and the ability to optimize such collaborations on 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 studies and clinical trials of DSG3-CAART, MuSK-CAART and CABA-201; the risk that the results observed with the similarly-designed construct employed in the recent *Nature Medicine* publication, including due to the dosing regimen, are not indicative of the results we seek to achieve with CABA-201; 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 volatile market and economic conditions; risks related to the impact of public health epidemics affecting countries or regions in which Cabaletta has operations or does business, such as COVID-19; Cabaletta's ability to retain and recognize the intended incentives conferred by Orphan Drug Designation and Fast Track Designation for its product candidates, as applicable; risks related to Cabaletta's ability to protect and maintain its intellectual property position; risks related to fostering and maintaining successful relationships with Cabaletta's collaboration and manufacturing partners; 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/or 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 and subsequent 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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