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Cabaletta Bio Receives Additional FDA Fast Track Designations for CABA-201 in Dermatomyositis and Systemic Sclerosis

– Second and third FDA Fast Track Designations for CABA-201, following the systemic lupus erythematosus (SLE) and lupus nephritis (LN) designation, providing the opportunity for expedited development and review of CABA-201 for the treatment of these autoimmune diseases –

PHILADELPHIA, Jan. 08, 2024 (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 separate Fast Track Designations to CABA-201, an investigational 4-1BB-containing fully human CD19-CAR T cell therapy, for the treatment of patients with dermatomyositis to improve disease activity and for the treatment of patients with systemic sclerosis (SSc) to improve associated organ dysfunction.

“The additional Fast Track Designations for CABA-201 in both dermatomyositis and systemic sclerosis, the second and third Fast Track Designations for CABA-201, provide the opportunity for expedited development and review of CABA-201 for the treatment of these autoimmune indications where there is a significant unmet need, despite currently available therapies,” said David J. Chang, M.D., Chief Medical Officer of Cabaletta. “We believe these designations potentially accelerate our ability to launch the first targeted, and potentially curative, cell therapy for autoimmune diseases driven by B cells. We look forward to continuing to leverage our research and translational insights along with our efficient trial designs in order to progress these programs forward for patients in need of better outcomes.”

CABA-201 is designed to deeply and transiently deplete CD19-positive B cells following a one-time infusion, which may enable an “immune system reset” with the potential for durable remission off therapy in patients with autoimmune diseases. To date, Cabaletta has received clearance from the FDA for Investigational New Drug (IND) applications for CABA-201 in multiple autoimmune conditions including systemic lupus erythematosus (SLE), myositis, SSc and generalized myasthenia gravis (gMG). Cabaletta is conducting four Phase 1/2 clinical trials with a total of nine cohorts that can advance simultaneously, employing a similar parallel cohort design and starting dose of 1 x 10^6 cells/kg without a dose escalation requirement.

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 Dermatomyositis
Dermatomyositis (DM) is an autoimmune disease that can lead to severe functional impairment that may be life-threatening despite best available standard of care. It is characterized by a skin rash along with muscle inflammation and weakness. Although the pathophysiology of DM is not well understood, it is thought to be a subtype of myositis that is driven by B cells. DM affects approximately 43,000 patients in the U.S. alone, and typically affects middle-aged individuals, particularly women. Current treatment typically involves medications to suppress the immune system and/or chronic intensive therapies such as intravenous immunoglobulin, or IVIg. Despite these therapies, a significant portion of DM patients have disease that remains refractory to existing medications.

About Systemic Sclerosis
SSc is a rare and potentially fatal chronic autoimmune disease characterized by progressive skin and internal organ fibrosis that can be life-threatening, including interstitial lung disease, pulmonary hypertension, and scleroderma renal crisis. Although the etiology of SSc is not well understood, the pathogenic role of autoantibodies and B cells in SSc provides a rationale for studying CAR T therapy in this population. SSc affects approximately 88,000 patients in the U.S., and typically affects middle-aged individuals, particularly women. Standard treatment options, which have modest effects, include generalized immunosuppressive agents or drugs targeted to specific symptomatic manifestations. Autologous hematopoietic stem cell transplant may provide some benefits in organ involvement, but carries significant risks, including mortality, infertility, and secondary autoimmune disease, limiting its potential to be applied broadly. Due to the lack of adequate treatments, the risk of mortality in systemic sclerosis remains high, with an average survival of approximately 12 years following 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 systemic lupus erythematosus, myositis, systemic sclerosis and generalized myasthenia gravis, 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 that offer deep and durable responses 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 by Fast Track Designations for CABA-201 in

patients with SLE and LN, dermatomyositis and SSc; Cabaletta'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 in patients across an increasing number of autoimmune diseases; Cabaletta's belief that it is making meaningful progress toward the development and potential launch of the first targeted, and perhaps curative, cellular therapies for patients with autoimmune diseases; the Company's advancement of separate Phase 1/2 clinical trials of CABA-201 in patients with SLE, myositis, SSc and gMG; Cabaletta's ability to leverage its research and translational insights; and the Company's expectations for the efficiency of the trial design for its Phase 1/2 clinical trials of CABA-201.

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: risks related to regulatory filings and potential clearance; 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 academic publications, 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 and public health crises; 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 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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