May 16, 2023

Cabaletta Bio Receives FDA Clearance of IND Application for CABA-201 for Treatment of Myositis

– Phase 1/2 trial for CABA-201 planned to initiate in patients with myositis at an initial dose that is equivalent to the dose used in a patient with myositis as reported in the recent Lancet Rheumatology *publication –*

– Second IND clearance for CABA-201, obtained within two months of first IND clearance in systemic lupus erythematosus (SLE) –

PHILADELPHIA, May 16, 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 Company's second Investigational New Drug (IND) application for CABA-201, a 4-1BB-containing fully human CD19-CAR T cell investigational therapy, has been cleared by the U.S. Food and Drug Administration (FDA) for a Phase 1/2 study in patients with active idiopathic inflammatory myopathy (IIM, or myositis). The Company plans to initiate a Phase 1/2 clinical trial of CABA-201 for the treatment of six patients with dermatomyositis (DM), six patients with anti-synthetase syndrome (ASyS), and six patients with immune-mediated necrotizing myopathy (IMNM), all in separate parallel cohorts. The initial dose for the trial, 1 x 10^6 cells/kg, was informed by preclinical data evaluating the binder in CABA-201 and the binder used in the CD19-CAR T construct administered to a patient with myositis in the recent *Lancet Rheumatology* publication.

"The clearance of our second IND application for CABA-201 within two months of the first IND clearance in SLE allows us to initiate a clinical trial in patients with myositis and underscores the efficiency of our organization along with the value of our experience in the development of cellular therapies for patients with autoimmune diseases," said Steven Nichtberger, M.D., Chief Executive Officer and Co-founder of Cabaletta. "Similar to our Phase 1/2 trial design in SLE, this clinical trial will include patients with several different subtypes of myositis where B cells may be involved in disease pathology. With an experienced team well-versed in conducting autoimmune-focused cell therapy trials, and a product candidate specifically engineered for patients with autoimmune diseases, we look forward to evaluating the potential for CABA-201 to change the treatment paradigm for patients with autoimmune diseases."

Myositis refers to a group of autoimmune diseases characterized by inflammation and muscle weakness. In some cases, myositis may also affect other organs and systems in the body, such as the lungs, heart, or skin. Myositis is classified into several subtypes based on the underlying immune mechanisms and clinical characteristics. Although the pathogenesis of myositis is not well understood, there are several subtypes thought to be driven by B cells, including dermatomyositis (DM), anti-synthetase syndrome (ASyS) and immune-mediated necrotizing myopathy (IMNM). These three subtypes impact approximately 66,000 patients

in the US alone, and typically affect middle-aged individuals, particularly women. All three subtypes can lead to severe functional impairment and may be life-threatening. 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 myositis patients have disease that remains refractory to existing medications.

The Phase 1/2 clinical trial will be an open-label study of CABA-201 in subjects with active myositis, including the subtypes of DM, ASyS and IMNM. Subjects will receive a one-time infusion of CABA-201 at a dose of 1.0 x 10^6 cells/kg, preceded by a standard preconditioning regimen of fludarabine and cyclophosphamide. Key inclusion criteria include patients between ages 18 to 65 (inclusive), evidence of active disease and disease activity despite prior or current treatment with standard of care treatments. Key exclusion criteria include cancer-associated myositis, significant lung or cardiac impairment, treatment with a B cell depleting agent within approximately six months or treatment with a biologic agent within approximately three months. As the second trial within Cabaletta's CARTA (Chimeric Antigen Receptor T cells for Autoimmunity) strategy, this study is intended to evaluate the potential ability of CABA-201 to transiently, but fully, eliminate B cells, enabling durable remissions via a "reset" of the immune system.

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 and myositis, 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 expectations regarding: 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 autoimmune diseases; the Company's plans to initiate (i) 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 and (ii) a Phase 1/2 clinical trial of CABA-201 in patients with myositis, including its anticipated progress, clinical trial design and ability to leverage its experience in autoimmune cell therapy; Cabaletta's ability to enroll the requisite number of patients, dose each dosing cohort in the intended manner in its Phase 1/2 clinical trials of CABA-201; 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; and the experience of our team in conducting autoimmune-focused cell therapy trials.

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 CABA-201; the risk that the results observed with the similarly-designed construct, including, but not limited to, dosing regimen, employed in the recent publications, including the *Lancet Rheumatology* publication, 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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