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U.S. Food and Drug Administration Grants Cabaletta Bio Fast Track Designation for MuSK-CAART

– Fast Track Designation granted to improve activities of daily living and muscle strength in patients with MuSK antibody-positive myasthenia gravis –

PHILADELPHIA, March 01, 2022 (GLOBE NEWSWIRE) -- Cabaletta Bio, Inc. (Nasdaq: CABA), a clinical-stage biotechnology company focused on the discovery and development of 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 MuSK-CAART, or muscle-specific kinase (MuSK) chimeric autoantibody receptor T (MuSK-CAART) cells, to improve activities of daily living and muscle strength in patients with MuSK antibody-positive myasthenia gravis. MuSK-CAART is being evaluated as a potential treatment for patients with MuSK-associated myasthenia gravis (MG).

Cabaletta's Investigational New Drug (IND) application was recently cleared by the FDA within the routine 30-day review period. Building on the clinical trial design and early data insights from Cabaletta's DesCAARTes™ trial evaluating DSG3-CAART cell therapy as a potential treatment for mucosal pemphigus vulgaris, the planned study to evaluate MuSK-CAART as a potential treatment for MuSK-associated MG starts with a 100 million cell dose cohort, bypassing the 20 million cell dose cohort that was used in the DesCAARTes™ trial, eliminates the dose fractionation regimen allowing for all dosing to be administered as a single infusion, and reduces the expected number of patients in each cohort from three to two patients in the absence of any dose limiting toxicities. Cabaletta plans to initiate a first-in-human clinical trial in 2022 for MuSK-CAART. The trial will be an open-label study consisting of two parts: 1) dose escalation to determine the maximum tolerated dose with two patients planned per cohort and 2) cohort expansion at the final selected dose; and is expected to enroll approximately 20 patients across multiple clinical sites throughout the United States.

"Anti-MuSK autoantibodies are observed in a subset of patients diagnosed with MG, and the limited treatment options for these patients underscore the need for a new and more effective standard of care," said David J. Chang, M.D., Chief Medical Officer of Cabaletta. "We believe the FDA's decision to grant Fast Track Designation highlights the need for a treatment capable of potentially delivering deep and durable responses for patients living with MuSK-associated MG. We look forward to initiating our first-in-human trial later this year."

MuSK-CAART is specifically designed to target B cells that differentiate into antibody secreting cells, which produce autoantibodies against muscle-specific kinase, a transmembrane protein found in muscle cells that is required for the formation and maintenance of the neuromuscular junction. In preclinical studies, MuSK-CAART has demonstrated *in vitro* selective and specific target engagement with no evidence of off-target

toxicity to date. Animal model studies suggest that MuSK-CAART is capable of *in vivo* target engagement through the elimination of anti-MuSK target cells.

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 MuSK-associated Myasthenia Gravis
MG is an autoimmune disease induced by autoantibodies targeting the neuromuscular junction (NMJ), which can lead to life-threatening muscle weakness. Generalized MG (gMG) is characterized by profound muscle weakness throughout the body, which may result in motor impairment, disabling fatigue, shortness of breath due to respiratory muscle weakness and episodes of respiratory failure. gMG affects approximately 50,000 to 80,000 patients in the United States. The majority of patients who develop gMG have autoantibodies against some part of the NMJ that are known to be pathogenic. 80% to 90% of patients with gMG have autoantibodies against the acetylcholine receptor detectable in their serum and are typically treated with acetylcholinesterase inhibitors as first-line therapy. Approximately 6% to 7.5% of patients with gMG have autoantibodies against muscle-specific kinase (MuSK), which is a different target on the surface of the muscle membrane. MuSK-associated MG patients typically do not respond to acetylcholinesterase inhibitors and are commonly treated with corticosteroids, generalized immunosuppressants, intravenous immunoglobulin, plasma exchange and rituximab. However, these methods require continuous treatment and have been associated with significant side effects and ongoing dependence on corticosteroids, which highlight the need for additional efficacious and safe treatments for patients diagnosed with MuSK-associated MG.

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, in combination with Cabaletta Bio's proprietary technology, has advanced a growing pipeline that currently includes potential treatments for patients with mucosal pemphigus vulgaris, MuSK-associated myasthenia gravis, PLA2R-associated membranous nephropathy, mucocutaneous pemphigus vulgaris and hemophilia A with FVIII alloantibodies. Cabaletta Bio's headquarters are located in Philadelphia, PA. For more information, visit www.cabalettabio.com and follow us on LinkedIn.

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: expectations regarding the intended incentives conferred by Fast Track Designation for MuSK-CAART to improve activities of daily living and muscle strength in patients with MuSK antibody-positive myasthenia gravis; the expectation that Cabaletta Bio may improve

outcomes for patients suffering from MuSK MG; plans to initiate patient dosing in an open-label Phase 1 clinical trial to evaluate MuSK-CAART safety and tolerability in MuSK MG patients in 2022; the ability of MuSK-CAART to target B cells that differentiate into antibody secreting cells, which produce autoantibodies against muscle-specific kinase; and the progress and results of its DesCAARTes™ Phase 1 trial, including Cabaletta Bio's ability to enroll the requisite number of patients, dose each dosing cohort in the intended manner, and advance the trial as planned.

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: Cabaletta's ability to retain and recognize the intended incentives conferred Fast Track Designation for MuSK-CAART to improve activities of daily living and muscle strength in patients with MuSK antibody-positive myasthenia gravis; the risk that signs of biologic activity may not inform long-term results; Cabaletta's ability to demonstrate sufficient evidence of safety, efficacy and tolerability in its preclinical and clinical trials of MuSK-CAART; 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 the impact of public health epidemics affecting countries or regions in which Cabaletta has operations or does business, such as COVID-19; risks related to Cabaletta's ability to protect and maintain its intellectual property position; uncertainties related to the initiation and conduct of studies and other development requirements for its product candidates; and the risk that the 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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