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# Cabaletta Bio Announces CABA-201, a Newly Designed CD19-Targeting CAR T Cell Therapy Engineered to Address a Broad Range of Autoimmune Diseases

*– Company has obtained exclusive worldwide license for a fully human CD19 binder with clinical tolerability data that support potential clinical development in autoimmune diseases –*

*– CABA-201 Investigational New Drug (IND) application planned for the first half of 2023 with initial clinical data expected by the first half of 2024, pending IND clearance –*

*– Clinical development plans being informed by exclusive translational research partnership with Georg Schett, M.D., senior author of the* Nature Medicine *publication reporting clinical and serologic disease remission and potential immune system reset in CD19-CAR T treated refractory systemic lupus erythematosus (SLE) patients and a global leader in the application of CD19-targeting cell therapies in autoimmunity –*

PHILADELPHIA, Oct. 11, 2022 (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 CABA-201, a newly designed, fully human CD19 chimeric antigen receptor (CAR) containing a 4-1BB co-stimulatory domain. Cabaletta has obtained an exclusive, worldwide license for the CD19 binder in CABA-201. The CD19 binder is integrated into a dual targeting CAR T therapy that has been evaluated in approximately 20 cancer patients to date in an investigator-initiated trial. We believe the tolerability data generated in these patients support clinical development in patients with autoimmune diseases. In addition, Cabaletta has established an exclusive translational research partnership with Dr. Georg Schett, a pioneer and global leader in the application of CD19-targeting cell therapies in autoimmunity. The collaboration is focused on generation of additional translational data to gain deeper understanding of the immunologic mechanisms of response and clinical insights from ongoing and continued clinical studies in multiple autoimmune disease indications. The construct utilized in these studies has a similar design to CABA-201, sharing the 4-1BB costimulatory domain and the binding region on the CD19 antigen with a fully human binder. With the addition of CABA-201 to its cell therapy pipeline, Cabaletta can potentially address a broad range of autoimmune diseases in indications such as SLE, rheumatoid arthritis, myositis and systemic sclerosis, among others where B cells contribute to disease pathogenesis.

“On the heels of the seminal publication in*Nature Medicine* last month reporting initial clinical activity and tolerability data from a 4-1BB-containing CD19-CAR T in patients with SLE who experienced durable drug-free clinical and serologic remission with the one-time therapy, we are excited to announce our new pipeline candidate, CABA-201. We believe CABA-201 is favorably designed for patients with autoimmune diseases given its fully human

CD19 binder and 4-1BB co-stimulatory domain. Our exclusive translational research partnership with Professor Schett, which is designed to leverage the deep experience and expertise of Cabaletta scientists in autoimmune cell therapy, has the potential to provide us with important and timely insights into patients enrolled in his breakthrough clinical studies," said Steven Nichtberger, M.D., Chief Executive Officer and Co-founder of Cabaletta. "We have a sufficient cash runway that will allow us to advance CABA-201 in parallel with the DesCAARTes™ and MusCAARTes™ trials employing our chimeric autoantibody receptor (CAAR) technology, with the potential to generate important clinical data readouts for each program. Accelerated by our team's proven experience in developing cell therapies for patients with autoimmune diseases in logistically complex trials, our next anticipated milestones for CABA-201 are an IND submission in the first half of 2023, and pending FDA clearance of the IND, initial clinical data by the first half of 2024. We believe CABA-201 has the potential to transform treatment of several common autoimmune diseases by providing clinical and serologic remission and a potential to reset the immune system, furthering our mission to develop therapies that deliver deep, durable, and potentially curative responses for patients with autoimmune diseases."

Data published by Professor Schett and his colleagues in*Nature Medicine* on September 15, 2022, demonstrate that a CD19-CAR T cell therapy with a 4-1BB co-stimulatory domain following lymphodepletion with fludarabine and cyclophosphamide induced persistent and deep clinical responses in five out of five patients with severe, refractory SLE, with up to 17 months of follow up. The safety profile demonstrated only mild cytokine release syndrome (CRS), with grade 1 CRS observed in three out of five patients, and no neurotoxicity (immune effector cell-associated neurotoxicity syndrome, or ICANS) of any grade observed. New B cells repopulated within five months of CD19-CAR T infusion in all patients, with no evidence of disease recurrence or autoantibodies following repopulation.

"There is significant unmet need in SLE and other autoimmune diseases, where we believe there is strong potential for CD19-targeting cell therapies to provide meaningful responses for patients. The team at Cabaletta has deep expertise in translational research relating to cell therapy in autoimmune patients, which will be complementary to my team's efforts. Together, through our exclusive translational research partnership, we can more efficiently address questions critical to advancing CD19-targeting cell therapy strategies for patients," stated Georg Schett, M.D., Professor and Head of the Department of Internal Medicine 3, and Vice President of Research, Friedrich-Alexander University, Erlangen-Nürnberg, Erlangen, Germany.

CABA-201 includes a fully human CD19 binder that was exclusively in-licensed from Nanjing IASO Biotherapeutics, Co., Ltd. (IASO Bio), which currently utilizes the binder in its CT120 product candidate, a 4-1BB-containing tandem CD19xCD22-CAR T cell therapy that has been evaluated in approximately 20 patients with promising tolerability data in an investigator-initiated trial. CT120 is currently in a Phase I clinical trial in China for non-Hodgkin's Lymphoma.

**Transaction Terms with IASO Bio**

Under the terms of the agreement, Cabaletta will receive an exclusive, worldwide license to IASO Bio's CD19 binder for use in autoimmune and alloimmune indications in humans. IASO Bio is eligible to receive up to $162 million in aggregate payments, including an upfront payment and payment upon the achievement of specified development and commercial

milestones, along with tiered mid-single digit royalties on future net sales for products that may result from this collaboration agreement.

**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 – encompassing chimeric antigen receptor T cells for autoimmunity (CARTA: CABA-201, a 4-1BB-containing CD19-CAR T) and Cabaletta Bio's proprietary chimeric autoantibody receptor T cells (CAART: multiple candidates including DSG3-CAART for mucosal pemphigus vulgaris, MuSK-CAART for MuSK myasthenia gravis) – provides multiple opportunities to treat broad and challenging autoimmune diseases. Cabaletta Bio's headquarters are located in Philadelphia, PA. For more information, visit www.cabalettabio.com and follow us on LinkedIn and Twitter.

**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 ability to capitalize on and potential benefits resulting from the translational research partnership with Professor Georg Schett and the exclusive license agreement with IASO Bio; the company's business plans and objectives; the timing of our planned submission of an investigational new drug application (IND) for CABA-201 to the FDA and generation of initial clinical data for CABA-201; statements regarding regulatory filings regarding its development programs; the expectation that Cabaletta Bio may improve outcomes for patients suffering from mPV, MG, or other autoimmune diseases; the progress and results of its DesCAARTes™ Phase 1 trial, including Cabaletta's ability to enroll the requisite number of patients, dose each dosing cohort in the intended manner, and progress the trial; 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 to accelerate Cabaletta's pipeline and develop meaningful therapies for patients, including in collaboration with academic and industry partners; and the anticipated contribution of the members of Cabaletta's executives to the company's operations and progress.

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; 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; the risk that any one or more of Cabaletta's product candidates will not be successfully developed or

commercialized; 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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