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Cabaletta Bio Announces Presentation of Data Supporting MuSK-CAART Development for the Treatment of the MuSK form of Myasthenia Gravis at the AAN 2020 Science Highlights Virtual Platform

In vivo *preclinical data show that Chimeric AutoAntibody Receptor (CAAR) T cells were able to specifically recognize and eliminate anti-MuSK antibody-expressing B cells while sparing control B cells*

PHILADELPHIA, May 18, 2020 (GLOBE NEWSWIRE) -- Cabaletta Bio, Inc. (Nasdaq: CABA), a clinical-stage biotechnology company focused on the discovery and development of engineered T cell therapies for patients with B cell-mediated autoimmune diseases, today announced that *in vivo* data demonstrating specific engagement and elimination of anti-muscle-specific tyrosine kinase (MuSK) antibody-expressing target cells by Chimeric AutoAntibody Receptor (CAAR) T cells was presented at the American Academy of Neurology (AAN) 2020 Science Highlights Virtual Platform by the laboratory of Aimee Payne, M.D., Ph.D., Associate Professor of Dermatology in the Perelman School of Medicine at the University of Pennsylvania and co-chair of the Scientific Advisory Board and co-founder at Cabaletta Bio. The research was performed in collaboration with Kevin O'Connor, Ph.D., Associate Professor of Neurology and Immunobiology at the Yale School of Medicine and an internationally recognized expert in the field of B cell-mediated autoimmune diseases, including myasthenia gravis, and was sponsored, in part, by Cabaletta Bio. Cabaletta Bio has exclusively licensed the MuSK CAAR T cell technology from and has developed the therapy in partnership with the University of Pennsylvania.

"This *in vivo* data in MuSK-associated myasthenia gravis (MuSK MG), a prototypical B cell-mediated autoimmune disease, is an important step to initiating a comprehensive IND-enabling program and supports the *in vitro* data previously presented at the American Neurological Association (ANA) Annual Meeting in late 2019," said Aimee Payne, M.D., Ph.D. "Patients who suffer from MuSK MG have very limited therapeutic options, primarily generalized immunosuppressants, which typically require chronic administration, and can cause significant side effects. There is a need for a therapy that specifically targets only the B cells causing the disease, while leaving normal B cells unaffected in patients suffering from MuSK MG."

Presentation details are as follows:

Title: MuSK chimeric autoantibody receptor (CAAR) T cells for antigen-specific cellular immunotherapy of myasthenia gravis

Abstract Number: 2769
Session: Clinical Trials and Therapeutics in Autoimmune Neurology
Link: www.AAN.com/2020science

In preclinical studies, MuSK CAAR T cells demonstrated *in vitro* cytotoxicity towards a B cell line expressing anti-MuSK antibodies, but no observed cytotoxicity when the anti-MuSK antibody was not expressed. In addition, MuSK CAAR T cells also targeted and eliminated a panel of B cells targeting different MuSK epitopes. In an *in vivo* mouse model, MuSK CAAR T cells, but not control CAAR T cells, showed biological activity by blocking the growth of B cell lines expressing an anti-MuSK antibody. This study demonstrated that MuSK CAAR T cells were able to deplete B cells expressing anti-MuSK antibodies.

Cabaletta plans to initiate Investigational New Drug (IND)-enabling studies for MuSK-CAART in 2020.

About Muscle-Specific Tyrosine Kinase Myasthenia Gravis
Muscle-specific Kinase Myasthenia Gravis (MuSK MG) is one form of Generalized MG (gMG) which is a chronic autoimmune disease induced by autoantibodies targeting the neuromuscular junction (NMJ), which can lead to profound and life-threatening muscle weakness throughout the body, resulting in motor impairment, disabling fatigue, shortness of breath due to respiratory muscle weakness and episodes of respiratory failure. Approximately 6% to 7.5% of the 65,000 to 70,000 gMG patients in the U.S. have autoantibodies against MuSK, which is a target on the surface of the muscle membrane. Compared to patients with the most common form of MG, acetylcholine receptor MG, MuSK MG patients have substantially fewer treatment options and more severe symptoms. Patients with MuSK MG are typically treated with corticosteroids in addition to one or more steroid-sparing broadly immunosuppressive agents.

About CAAR T Cell Therapy
Chimeric AutoAntibody Receptor (CAAR) T cells are designed to selectively bind and eliminate only disease-causing B cells, while sparing the normal B cells that are essential for human health. CAAR T cells are based on chimeric antigen receptor (CAR) T cell technology. While CAR T cells typically contain a CD19-targeting molecule, CAAR T cells express an autoantibody-targeted antigen on their surface. The co-stimulatory domain and the signaling domain of both a CAR T cell and a CAAR T cell carry out the same activation and cytotoxic functions. Thus, Cabaletta's CAARs are designed to direct the patient's T cells to kill only the pathogenic cells that express disease-causing autoantibodies on their surface, potentially leading to complete and durable remission of disease while sparing all other B cell populations that provide beneficial immunity from infection.

About Cabaletta Bio
Cabaletta Bio is a clinical-stage biotechnology company focused on the discovery and development of engineered T cell therapies for patients with B cell-mediated autoimmune diseases. The Cabaletta Approach to selective B cell Ablation (CABA) platform, in combination with Cabaletta's proprietary technology, utilizes Chimeric AutoAntibody Receptor (CAAR) T cells that are designed to selectively bind and eliminate only specific autoantibody-producing B cells while sparing normal antibody-producing B cells, which are essential for human health. The Company's lead product candidate, DSG3-CAART, is in development as a potential treatment for a prototypical B cell-mediated autoimmune disease, mucosal pemphigus vulgaris. For more information, visit www.cabalettabio.com.

Forward-Looking Statements
This press release contains forward-looking statements within the meaning of the Private Securities Litigation Reform Act of 1995, as amended, including, without limitation, express or implied statements regarding our expectations on the preliminary results from *in vivo* data in MuSK MG; the timing of our planned initiation of investigational new drug (IND) enabling studies for MuSK-CAART, our expectations regarding the results from our IND-enabling studies for MuSK-CAART and the FDA's review of the results therefrom, our expectations regarding the ability of CAAR T cells to treat MuSK-CAART, the expected timing and progress of preclinical studies and clinical trials for our other product candidates based on our CABA platform, and competition from other biotechnology companies, and our guidance regarding the therapeutic potential and clinical benefits of our product candidates, as well as the potential patient population that may be addressed. The words "may," "will," "could," "would," "should," "expect," "plan," "anticipate," "intend," "believe," "estimate," "predict," "project," "potential," "continue," "target" and similar expressions are intended to identify forward-looking statements, although not all forward-looking statements contain these identifying words. Any forward-looking statements in this press release are based on management's current expectations and beliefs and are subject to a number of risks, uncertainties and important factors that may cause actual events or results to differ materially from those expressed or implied by any forward-looking statements contained in this press release, including, without limitation, risks and uncertainties related to the delay of any current or planned preclinical studies or clinical trials or the development of our product candidates, our ability to successfully demonstrate the efficacy and safety of our drug candidates including in later-stage studies and trials, the preclinical and clinical results for our product candidates, which may not support further development of such product candidates, actions of regulatory agencies, any or all of which may affect the initiation, timing and progress of preclinical studies, clinical trials and regulatory development. We caution you not to place undue reliance on any forward-looking statements, which speak only as of the date they are made. We disclaim any obligation to publicly update or revise any such statements to reflect any change in expectations or in events, conditions or circumstances on which any such statements may be based, or that may affect the likelihood that actual results will differ from those set forth in the forward-looking statements. Any forward-looking statements contained in this press release represent our views only as of the date hereof and should not be relied upon as representing its views as of any subsequent date. We explicitly disclaim any obligation to update any forward-looking statements.

Editor's Note: Dr. Payne is a University of Pennsylvania faculty member and holds an equity stake in the Company, and the University of Pennsylvania is an equity holder and investor in the Company. In addition, both the University of Pennsylvania and the inventors of the licensed technology may receive additional financial benefits under the license in the future.

Contacts:
Anup Marda
Chief Financial Officer
investors@cabalettabio.com

Investors:
Sarah McCabe
Stern Investor Relations, Inc.

sarah.mccabe@sternir.com

Source: Cabaletta Bio