October 7, 2019

Cabaletta Bio Announces Presentation of Preclinical Data Showing Specific CAAR T Activity against Anti-MuSK Expressing B Cells

Study to be presented by the laboratory of University of Pennsylvania professor Aimee Payne, M.D., Ph.D., Cabaletta co-founder and Scientific Advisory Board co-chair, as a highlighted abstract at the American Neurological Association (ANA) 2019 Annual Meeting in St. Louis

PHILADELPHIA, Oct. 07, 2019 (GLOBE NEWSWIRE) -- Cabaletta Bio, Inc., 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 a preclinical *in vitro* study demonstrating specific killing of anti-muscle-specific tyrosine kinase (MuSK) antibody-expressing target cells by Chimeric AutoAntibody Receptor T (CAAR T) cells. The ability to target antibody-expressing cells with a CAAR T cell may provide a new approach to the treatment of MuSK-associated myasthenia gravis (MG). The study has been featured by and will be presented at the American Neurological Association (ANA) 2019 Annual Meeting, October 13 to 15, in St. Louis.

“MuSK myasthenia gravis is a serious antibody-mediated disease with limited therapeutic options. The pathogenic antibodies that cause MuSK MG are well-defined suggesting that this disease is an ideal candidate for our CAAR T approach,” said Aimee Payne, M.D., Ph.D., co-founder and co-chair of the Scientific Advisory Board at Cabaletta Bio and the Albert M. Kligman Associate Professor of Dermatology in the Perelman School of Medicine at the University of Pennsylvania and a member of Penn’s Abramson Cancer Center. “The preclinical data establish the *in vitro* function of a MuSK CAAR T cell against multiple epitopes on the target B cells.”

Gwendolyn Binder, Ph.D., EVP Science and Technology at Cabaletta Bio, added, “This research from our co-founder and collaborators at the University of Pennsylvania is expected to form the basis of our second cell therapy product candidate, MuSK-CAART. As part of our cooperative mission with our cofounders, we look forward to continuing to advance this pipeline program and potential therapy for patients with MuSK MG.”

Presentation and poster session details:

Title:	Antigen-Specific B Cell Depletion for Myasthenia Gravis with Chimeric Autoantibody Receptor (CAAR) T Cells
Authors:	Sangwook Oh, Ph.D. (Presenter), Kevin O’Connor, PhD, Aimee S. Payne, MD, PhD
Abstract #:	M101
Poster:	Monday, October 14, 2019, at 5:00 p.m. CDT
Location:	Majestic E-H

Oral Presentation:	Tuesday, October 15, 2019, at 10:29 a.m. CDT
Location:	Majestic D

MuSK-CAART is the second product candidate that has emerged from the Cabaletta Approach to selective B cell Ablation (CABA™) platform. The Company has received IND clearance for its lead product candidate, desmoglein 3 CAART, or DSG3-CAART, for patients with mucosal Pemphigus Vulgaris (mPV), which is expected to enter a Phase 1 clinical trial in 2020. The company has additional discovery programs derived from the CABA Platform for treating patients with mucocutaneous PV and for hemophilia A patients who have developed alloantibodies to Factor VIII.

CAAR T cell therapy has the potential to target the pathogenic B cells producing autoantibodies to MuSK while sparing healthy B cells. For the preclinical study being presented, investigators generated CAARs expressing MuSK as an extracellular decoy on the surface of T cells. These CAARs were observed to direct T cell cytotoxicity toward B cells expressing anti-MuSK antibodies resulting in their destruction. Based on this initial *in vitro* study, CAAR T cells may represent a novel therapeutic approach for targeted B cell depletion in MuSK MG.

Myasthenia gravis (MG) is an autoimmune disease induced by autoantibodies targeting the neuromuscular junction (NMJ), which can lead to life-threatening muscle weakness. The most common form, accounting for 85% of MG cases, involves B cell-produced autoantibodies that target the acetylcholine receptor and destroy the NMJ. A rarer but more severe subtype with fewer treatment options, MuSK MG, affects 6 to 7.5% of the MG population. MuSK is a muscle receptor tyrosine kinase that is essential for neuromuscular junction development and function. Current therapeutic strategies for MG involve generalized immune suppression to reduce antibody production, which has significant risks and has been observed to cause lethal infections in certain patients.

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 the chimeric antigen receptor (CAR) T cell technology developed at the University of Pennsylvania. 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 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. Cabaletta's lead product candidate is based on the Chimeric Antigen Receptor (CAR) T cell technology developed at the University of Pennsylvania (Penn) that resulted in one of the first commercially-available CAR T cell products for the treatment of B cell cancers. Cabaletta was founded by Penn physician/scientists Michael Milone, M.D., Ph.D., and Aimee Payne, M.D., Ph.D., who serve as co-chairs of Cabaletta's Scientific Advisory Board, and Steven Nichtberger, M.D., CEO of Cabaletta. Cabaletta has an exclusive global licensing agreement and multiple sponsored research agreements with the University of Pennsylvania to develop the CAAR T technology to treat B cell-mediated autoimmune diseases. The Company's lead product candidate is being studied as a potential treatment for a prototypical B cell-mediated autoimmune disease, mucosal pemphigus vulgaris (mPV). For more information, visit www.cabalettabio.com.

Cautionary Note Regarding 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, those regarding the planned timing, enrollment and results for our Phase 1 clinical trial for DSG3-CAART for the treatment of mPV, our expectations regarding the ability of MuSK-CAART to treat MuSK MG, the planned timing for additional IND applications, the expected timing and progress of preclinical studies and clinical trials for our other product candidates based on our CABA platform, our ability to meet the objectives of our planned preclinical studies and clinical trials and demonstrate the safety and efficacy of our product candidates, our ability to fund the development of our CABA platform product candidates, the development of our CABA platform product candidates and their therapeutic potential, whether and when, if at all, our CABA platform product candidates will receive approval from the FDA and for which, if any, indications, and competition from other biotechnology companies. 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, including MuSK-CAART, 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: Drs. Payne, Milone and Nichtberger are University of Pennsylvania faculty members and hold equity stakes in the Company, and the University of Pennsylvania is an equity holder and investor in the Company. In addition, both Penn and the inventors of the licensed technology may receive additional financial benefits under the license in the future.

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