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Cabaletta Bio Reports Second Quarter 2022 Financial Results and Provides Business Update

PHILADELPHIA, Aug. 11, 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 reported financial results for the second quarter ended June 30, 2022, and provided a business update.

“The DesCAARTes™ trial is continuing to advance through additional cohorts, including higher doses as well as a planned combination cohort with intravenous immunoglobulin and cyclophosphamide administered prior to DSG3-CAART infusion, which are expected to start dosing following cohort A5. We also expect to present 6 month clinical and translational data from cohort A4 as well as 28-day safety data from cohort A5 at the upcoming European Association of Dermatology and Venereology Congress next month,” said Steven Nichtberger, M.D., Chief Executive Officer and Co-founder of Cabaletta. “With cash on hand to fund operations through the first quarter of 2024, the goal of our autoimmune-focused pipeline is to achieve deep, durable and perhaps curative outcomes for patients. We are confident that we are well-positioned to build on our progress to date and deliver long-term value to patients and our other key stakeholders.”

Pipeline Highlights and Anticipated Upcoming Milestones

DSG3-CAART: Desmoglein 3 chimeric autoantibody receptor T (DSG3-CAART) cells as a potential treatment for patients with mucosal pemphigus vulgaris (mPV).

- **Presented updated interim clinical data from the ongoing DesCAARTes™ trial at ASGCT and SID Annual Meetings:** In May 2022, Cabaletta presented updated clinical and translational data through 6 months of follow-up in cohorts A1 through A3, safety data through 3 months and persistence data through 1 month of follow-up in cohorts A1 through A4 from the DesCAARTes™ trial at the American Society of Gene & Cell Therapy (ASGCT) 25th Annual Meeting and Society For Investigative Dermatology (SID) 2022 Annual Meeting. The updated interim data demonstrated that DSG3-CAART had a favorable safety profile with no dose limiting toxicities or cytokine release syndrome of any grade through cohort A4 and that a dose dependent increase in DSG3-CAART persistence was observed through day 29 in cohorts A1 through A4.

- **Additional data from the DesCAARTes™ trial anticipated at the 31st European Dermatology and Venereology (EADV) Congress:** Cabaletta plans to disclose 6 month clinical and translational data for cohort A4 and 28-day safety data for cohort A5 at the 31st EADV Congress, which is being held in Milan, Italy from September 7-10, 2022.

- **Upcoming cohorts designed to maximize DSG3-CAART exposure in vivo** Two

additional dose cohorts are planned after cohort A5: A6m (multi-dose regimen at 10 to 15 billion cells) and a combination cohort (2.5 billion cells in addition to patient pre-treatment with intravenous immunoglobulin [IVIg] and cyclophosphamide). The prioritization of cohorts following cohort A5 (e.g. A6m or combination) is subject to evaluation of emerging data and finalization of our protocol, as applicable. Cohort A5e (enhanced manufacturing process at 5.0 to 7.5 billion cells) is no longer planned to occur immediately after cohort A5.

MuSK-CAART: Muscle-specific kinase (MuSK) chimeric autoantibody receptor T (MuSK-CAART) cells as a potential treatment for patients with MuSK-associated myasthenia gravis.

- **Presented preclinical data supporting planned clinical development:** In May 2022, Cabaletta presented preclinical safety and activity studies supporting precision engineered T-cell therapy for MuSK myasthenia gravis at American Association of Immunologists IMMUNOLOGY2022™, 14th MGFA International Conference On Myasthenia And Related Disorders and American Society of Gene & Cell Therapy 25th Annual Meeting. The preclinical data demonstrated that MuSK-CAART eliminated anti-MuSK target cells in an animal model where CART19 cells were a positive control. The preclinical data suggest that MuSK-CAART demonstrated specific *in vivo* target engagement and support its progression into clinical evaluation.

- **Clinical Trial Application for MusCAARTes™ trial accepted by Health Canada:** In June 2022, Health Canada issued a No Objection Letter (NOL) in response to a Clinical Trial Application for the MusCAARTes™ trial submitted by Cabaletta. The NOL allows for Cabaletta to activate clinical trial sites and pursue patient enrollment for the MusCAARTes™ trial in Canada. The receipt of the NOL from Health Canada follows the clearance of an Investigational New Drug (IND) application submitted by Cabaletta to the FDA for the MusCAARTes™ trial, which was cleared within the routine 30-day review period. MuSK-CAART was granted Fast Track Designation in March 2022.

- **First-in-human trial planned to initiate in 2022:** The trial will be an open-label study consisting of two parts: (i) an accelerated dose escalation phase with a “2+4” dosing scheme designed to determine the maximum tolerated dose, with four additional patients added at the highest selected dose and (ii) a cohort expansion phase at the final selected dose. The trial will incorporate insights and enhancements supported by data from the DesCAARTes™ trial, including the ability to start at a higher initial dose. The trial is expected to enroll approximately 24 patients across multiple clinical sites throughout the United States and Canada.

Upcoming Events

Cabaletta will participate in the following upcoming investor conferences:

- Morgan Stanley 20th Annual Global Healthcare Conference, which is being held in New York, NY from September 12-14, 2022.

- H.C. Wainwright 24th Annual Global Investment Conference, which is being held virtually and in person in New York, NY from September 12-14, 2022.

Second Quarter 2022 Financial Results

- Research and development expenses were $9.5 million for the three months ended June 30, 2022, compared to $7.9 million for the same period in 2021.

- General and administrative expenses were $3.5 million for the three months ended June 30, 2022, compared to $3.3 million for the same period in 2021.

- As of June 30, 2022, Cabaletta had cash, cash equivalents and investments of $96.8 million, compared to $122.2 million as of December 31, 2021.

Based on updated forecasting, the Company expects that its cash, cash equivalents and investments as of June 30, 2022 will enable it to fund its operating plan through the first quarter of 2024.

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: Cabaletta's ability to grow and advance its autoimmune-focused and preclinical pipeline; the progress and results of its DesCAARTes™ Phase 1 trial and planned MusCAARTes™ trial, including its ability to enroll the requisite number of patients, dose each dosing cohort in the intended manner, and progress the trial; the significance and impact around the clinical and translational data updates from cohorts A1 through A3 of the DesCAARTes™ trial; the expected timing and significance of the announcement of 28-day safety for cohort A5 and clinical and translational data for cohort A4 at the 31st EADV Congress in September 2022; the expectation that Cabaletta may improve outcomes for patients suffering from mPV; Cabaletta's ability to escalate dosing as high as 10 to 15 billion cells in a planned future cohort, initiate dosing in a combination cohort or otherwise; Cabaletta's plans to implement a pre-treatment regimen; Cabaletta's ability to advance dose escalation in the DesCAARTes™ Phase 1 trial at the current dose ranges for the current cohorts and any projected potential dose ranges for future cohorts, and to optimize its targeted cell therapy; Cabaletta's ability to evaluate, and the potential significance of, the relationship between DSG3-CAART persistence and potential clinical responses in patients with mPV; 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 effectiveness and timing of product candidates that Cabaletta may develop, including in collaboration with academic partners; presentation

of additional data at upcoming scientific conferences, and other preclinical data; expectations regarding the design, implementation, timing and success of its current and planned clinical trials and the successful completion of nonclinical studies; planned potential timing and advancement of its preclinical studies and clinical trials and related regulatory submissions; ability to optimize the impact of its collaborations on its development programs; use of capital, expenses, future accumulated deficit and other financial results in the future; and ability to fund operations through the first quarter of 2024.

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 and clinical trials of DSG3-CAART and MuSK-CAART; the risk that persistence observed with effective CART-19 oncology studies in combination with lymphodepletion is not indicative of, or applicable to, clinical responses in patients with mPV; 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, such as the ongoing COVID-19 pandemic, affecting countries or regions in which we have operations or do business; Cabaletta's ability to retain and recognize the intended incentives conferred by Orphan Drug Designation and Fast Track Designation for DSG3-CAART for improving healing of mucosal blisters in patients with mucosal pemphigus vulgaris; Cabaletta's ability to demonstrate sufficient evidence of safety, efficacy and tolerability in its preclinical and clinical trials of DSG3-CAART and MuSK-CAART; risks related to fostering and maintaining successful relationships with Cabaletta's manufacturing partners; 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 and 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 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.

CABALETTA BIO, INC.
SELECTED FINANCIAL DATA
(unaudited; in thousands, except share and per share data)

Statements of Operations

	Three Months Ended June 30,	Six Months Ended June 30,

	2022	2021	2022	2021
	unaudited		unaudited	
Operating expenses:				
Research and development	$ 9,514	$ 7,850	$ 18,684	$ 14,406
General and administrative	3,546	3,295	7,375	6,451
Total operating expenses	13,060	11,145	26,059	20,857
Loss from operations	(13,060)	(11,145)	(26,059)	(20,857)
Other income:				
Interest income	150	6	203	16
Net loss	(12,910)	(11,139)	(25,856)	(20,841)
Net loss per share of voting and non-voting common stock, basic and diluted	$ (0.45)	$ (0.45)	$ (0.89)	$ (0.86)

Selected Balance Sheet Data

	June 30, 2022	December 31, 2021
	(unaudited)	
Cash, cash equivalents and investments	$ 96,806	$ 122,222
Total assets	102,016	126,336
Total liabilities	6,486	8,380
Total stockholders' equity	95,530	117,956

Contacts:

Anup Marda
Chief Financial Officer
investors@cabalettabio.com

Sarah McCabe
Stern Investor Relations, Inc.
212-362-1200
sarah.mccabe@sternir.com

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