Cabaletta Bio

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

PHILADELPHIA, Nov. 10, 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 reported financial results for the third quarter ended September 30, 2022, and provided a business update.

“Our recently announced product candidate, CABA-201, a proprietary, fully human CD19-chimeric antigen receptor (CAR) T construct containing a 4-1BB co-stimulatory domain, was purposefully designed to be similar to the 4-1BB containing CD19-CAR T construct employed in the recent *Nature Medicine* publication, which demonstrated profound clinical and serologic responses with a generally favorable clinical tolerability profile in five of five systemic lupus erythematosus patients with a single administration. Based on our deep experience with discovery, development and regulatory interactions for CAAR T products in patients with autoimmune diseases, we believe we can efficiently and effectively evaluate CABA-201’s potential across a broad range of autoimmune diseases. We are planning to submit an IND application to the FDA in the first half of 2023, and expect initial clinical data, subject to IND clearance by the FDA, by the first half of 2024,” said Steven Nichtberger, M.D., Chief Executive Officer and Co-founder of Cabaletta. “We also continue to progress our CAART product candidate portfolio, including prioritizing the enrollment of the combination sub-study in the DesCAARTes™ trial for DSG3-CAART, with 1-month safety and persistence data expected in the first quarter of 2023, and continuing preparations to initiate the MusCAARTes™ trial for MuSK-CAART in the fourth quarter of 2022.”

Third Quarter 2022 and Recent Operational Highlights and Upcoming Milestones

Chimeric Antigen Receptor T cells for Autoimmunity (CARTA) Platform

CABA-201: Autologous, engineered T cells with a chimeric antigen receptor containing a fully human CD19 binder and a 4-1BB co-stimulatory domain as a potential treatment for a broad range of autoimmune diseases in indications such as systemic lupus erythematosus (SLE), rheumatoid arthritis, myositis and systemic sclerosis, among others where B cells contribute to disease pathogenesis.

- **Obtained exclusive, worldwide license from Nanjing IASO Biotherapeutics, Co., Ltd. (IASO Bio) for binder to be used in new product candidate CABA-201:** In October 2022, Cabaletta obtained the CD19 binder for its new product candidate, CABA-201, through an exclusive, worldwide license with IASO Bio. This CD19 binder is separately being used as part of a dual targeting CAR T therapy that has been evaluated in approximately 20 cancer patients to date in an investigator-initiated trial. Tolerability data generated in these patients support further clinical development in patients with autoimmune diseases.

- **Established an exclusive translational research partnership with Georg Schett, M.D.:** In October 2022, Cabaletta signed an exclusive translational research partnership with Dr. Georg Schett, a pioneer and global leader in the application of CD19-targeting cell therapies in autoimmunity and senior author of the September 2022 *Nature Medicine* publication demonstrating the potential for CD19-CAR T therapy to reset the immune system in five of five patients with refractory SLE. The Company’s collaboration is focused on generating additional translational data to gain a deeper understanding of the immunologic mechanisms of response and clinical insights from ongoing and continued clinical studies in multiple autoimmune disease indications. The clinical development of CABA-201 will be informed by this exclusive translational research partnership.

- **Investigational New Drug (IND) application submission planned for 1H 2023:** The Company anticipates submitting an IND to the FDA for CABA-201 in the first half of 2023. Subject to FDA clearance of the IND, the Company expects to report initial clinical data by the first half of 2024.

Chimeric AutoAntibody Receptor T (CAART) Cells Platform

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

- **Presented new interim data from the DesCAARTes™ Phase 1 Trial at the 31st European Academy of Dermatology and Venereology (EADV) Congress and the 29th Annual European Society of Gene & Cell Therapy Congress:** In September 2022 and October 2022, Cabaletta presented updated data supporting a favorable safety profile of DSG3-CAART with no dose-limiting toxicities, and one grade 1 cytokine release syndrome, through cohort A5, which provided a rationale to prioritize the enrollment of the cohort in the combination sub-study (2.5 billion cells in addition to patient pre-treatment with intravenous immunoglobulin [IVIg] and cyclophosphamide), with the goal to address possible cytokine and autoantibody effects on CAART activity. The Company anticipates 1-month safety and persistence data for the combination cohort in the first quarter of 2023.

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

- **Granted Orphan Drug Designation (ODD) by FDA:** In October 2022, the FDA granted ODD to MuSK-CAART for the treatment of muscle-specific tyrosine kinase myasthenia gravis. The FDA grants ODD to drugs or biologics intended to treat or prevent rare diseases or conditions that affect fewer than 200,000 individuals in the United States. This designation qualifies Cabaletta for certain incentives, which may include partial tax credit for clinical trial expenditures, waived user fees and potential eligibility for seven years of marketing exclusivity.

- **First-in-human trial to initiate in the fourth quarter of 2022:** Cabaletta remains on track to initiate the MusCAARTes™ trial for MuSK-CAART in the fourth quarter of 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 and an earlier initiation of the combination cohort, where patients are planned to be pre-treated with cyclophosphamide. The trial is expected to enroll patients across multiple clinical sites throughout the United States and Canada. Based on current clinical expectations, the Company expects 6-month data for the combination cohort of the MusCAARTes™ trial for MuSK-CAART in the first half of 2024.

Upcoming Events

Cabaletta will participate in the upcoming 5th Annual Evercore ISI HealthCONx Conference, which is being held virtually from November 29 – December 1, 2022.

Third Quarter 2022 Financial Results

- Research and development expenses were $8.2 million for the three months ended September 30, 2022, compared to $8.2 million for the same period in 2021.
- General and administrative expenses were $3.6 million for the three months ended September 30, 2022, compared to $3.4 million for the same period in 2021.
- As of September 30, 2022, Cabaletta had cash, cash equivalents and investments of $85.9 million, compared to $122.2 million as of December 31, 2021.

The Company expects that its cash, cash equivalents and investments as of September 30, 2022, will enable it to fund its operating plan through the second 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 – 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.

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 IND application for CABA-201 to the FDA and generation of initial clinical data for CABA-201; statements regarding regulatory filings for its development programs, including the planned timing of such regulatory filings and potential review by such regulatory authorities; 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 retain and recognize the intended incentives conferred by Orphan Drug Designation for MuSK-CAART for the treatment of muscle-specific tyrosine kinase myasthenia gravis; the ability to accelerate Cabaletta’s pipeline and develop meaningful therapies for patients, including in collaboration with academic and industry partners and the ability to optimize such collaborations on its development programs; use of capital, expenses, future accumulated deficit and other financial results in the future; ability to fund operations through the second quarter of 2024; 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 DSG3-CAART, MuSK-CAART and CABA-201; the risk that the results observed with the similarly-designed construct employed in the recent *Nature Medicine* publication are not indicative of the results we seek to achieve with 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 volatile market and economic conditions; 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; Cabaletta’s ability to retain and recognize the intended incentives conferred by Orphan Drug Designation and Fast Track Designation for its product candidates, as applicable; risks related to Cabaletta’s ability to protect and maintain its intellectual property position; risks related to fostering and maintaining successful relationships with Cabaletta’s collaboration and manufacturing partners; 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/or 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 September 30,		Nine Months Ended September 30,	
	2022	2021	2022	2021
	unaudited		unaudited	
Operating expenses:				
Research and development	$ 8,216	$ 8,169	$ 26,900	$ 22,575
General and administrative	3,562	3,394	10,937	9,845
Total operating expenses	11,778	11,563	37,837	32,420
Loss from operations	(11,778)	(11,563)	(37,837)	(32,420)
Other income:				
Interest income	351	3	554	19
Net loss	(11,427)	(11,560)	(37,283)	(32,401)
Net loss per share of voting and non-voting common stock, basic and diluted	$ (0.39)	$ (0.45)	$ (1.29)	$ (1.31)

Selected Balance Sheet Data

	September 30, 2022	December 31, 2021
	(unaudited)	
Cash, cash equivalents and investments	$ 85,895	$ 122,222
Total assets	91,675	126,336
Total liabilities	5,801	8,380
Total stockholders' equity	85,874	117,956

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