March 21, 2024

Cabaletta Bio Reports Fourth Quarter and Full Year 2023 Financial Results and Provides Business Update

– First patient dosed with CABA-201 in the RESET™ (REstoring SElf-Tolerance) clinical trial program –

– Initial clinical data from each of the first patients in the RESET-Myositis™ and RESET-SLE™ trials anticipated in 1H24; longer term follow-up on these and additional patients to be reported in 2H24 –

– RESET-SSc™ (systemic sclerosis) and RESET-MG™ trials initial clinical data anticipated to be reported in 2H24 –

– Rare Pediatric Disease designation granted by FDA for CABA-201 in juvenile dermatomyositis –

– Cash runway into 1H26 with cash, cash equivalents and short-term investments of $241.2 million as of December 31, 2023 –

PHILADELPHIA, March 21, 2024 (GLOBE NEWSWIRE) -- Cabaletta Bio, Inc. (Nasdaq: CABA), a clinical-stage biotechnology company focused on developing and launching the first curative targeted cell therapies designed specifically for patients with autoimmune diseases, today reported financial results for the fourth quarter and full year ended December 31, 2023, and provided a business update.

“Throughout the past year, we set the foundation to enable an efficient development strategy for CABA-201 across a broad range of autoimmune diseases. Clinical sites across the United States are actively recruiting for our myositis and SLE trials, and the first patient has been dosed with no CRS or ICANS of any grade observed as of 21 days following CABA-201 infusion. We look forward to building on this momentum with expansion into additional clinical sites and to delivering data across the RESET program in 2024,” said Steven Nichtberger, M.D., Chief Executive Officer and Co-founder of Cabaletta. “By advancing individual, company-sponsored trials for each autoimmune disease without the need for an initial dose escalation study, we believe we have an accelerated path to initiate discussions with the FDA on registrational cohorts and/or studies following treatment of six patients in any one of the nine cohorts in our current RESET clinical trial program. Our commitment to exploring the broad potential of CABA-201 for patients is further demonstrated by the recently granted Rare Pediatric Disease designation in juvenile dermatomyositis, an indication in which there are no FDA approved therapies. Based on our strong balance sheet and a differentiated CABA-201 development strategy, we believe we are well positioned to develop and launch the first targeted, and perhaps curative, cell therapy specifically designed for patients with autoimmune diseases.”

Recent Operational Highlights and Upcoming Anticipated Milestones

Chimeric Antigen Receptor T cells for Autoimmunity (CARTA) Strategy

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 across multiple therapeutic portfolios where B cells contribute to the initiation and/or maintenance of disease.

Rheumatology Portfolio

- **CABA-201 in myositis (idiopathic inflammatory myopathies)**
  - The first patient has been dosed in the Phase 1/2 RESET-Myositis trial with no CRS (cytokine release syndrome) or ICANS (immune effector cell-associated neurotoxicity syndrome) of any grade observed for the first 21 days of a 28-day dose-limiting toxicity observation window following administration.
  - Enrollment in the Phase 1/2 RESET-Myositis trial is ongoing across multiple sites in the U.S.

- **CABA-201 in systemic lupus erythematosus (SLE)**
  - Enrollment is underway in the Phase 1/2 RESET-SLE trial, which is being conducted across multiple sites in the U.S.
  - In March 2024, Health Canada issued a No Objection Letter (NOL) in response to a Clinical Trial Application for the RESET-SLE trial submitted by Cabaletta. The NOL allows for Cabaletta to begin the process to activate clinical trial sites and pursue patient enrollment for the RESET-SLE trial in Canada.

- **CABA-201 in systemic sclerosis (SSc)**
  - In October 2023, Cabaletta received Investigational New Drug (IND) application clearance from the U.S. Food and Drug Administration (FDA) for the Phase 1/2 RESET-SSc trial.
  - Cabaletta anticipates reporting initial clinical data from the Phase 1/2 RESET-SSc trial in the second half of 2024.

- **CABA-201 regulatory designation updates**
  - In January 2024, Cabaletta announced that CABA-201 was granted Fast Track Designations by the FDA for the treatment of patients with dermatomyositis to improve disease activity and for the treatment of patients with SSc to improve associated organ dysfunction.
  - In February 2024 and March 2024, Cabaletta announced that CABA-201 was granted Orphan Drug Designation by the FDA for the treatment of idiopathic inflammatory myopathies (IIM, or myositis) and for the treatment of systemic sclerosis, respectively.
  - Cabaletta has been recently granted Rare Pediatric Disease Designation for the treatment of juvenile dermatomyositis. This designation may allow the Company to be eligible for a priority review voucher, assuming reauthorization of the program by the U.S. federal government, for a subsequent marketing application at the time of marketing approval for CABA-201.

Neurology Portfolio

- **CABA-201 in generalized myasthenia gravis (gMG)**

    - In November 2023, Cabaletta announced that its IND application for CABA-201 was allowed to proceed by the FDA for the Phase 1/2 RESET-MG trial.
    - Cabaletta anticipates reporting initial clinical data from the Phase 1/2 RESET-MG trial in the second half of 2024.

External Scientific Presentations

- In February 2024, Cabaletta presented a poster presentation on new preclinical CABA-201 specificity and activity data for treatment-resistant autoimmune disease at the 2024 Tandem Meetings | Transplantation & Cellular Therapy (TCT) Meetings of ASTCT® (American Society for Transplantation and Cellular Therapy) and CIBMTR® (Center for International Blood and Marrow Transplant Research).

- In March 2024, Cabaletta presented a poster presentation on new preclinical specificity and activity data in treatment resistant myositis at the 5th Global Conference on Myositis. In addition, David J. Chang, M.D., Chief Medical Officer of Cabaletta, moderated a symposium featuring Carl H. June, M.D., and Rohit Aggarwal, M.D., titled "The Next Frontier for CAR T Cells: Autoimmune Disease" on March 14, 2024.

Chimeric AutoAntibody Receptor T (CAART) cells Strategy

- **DSG3-CAART:** Cabaletta is evaluating desmoglein 3 chimeric autoantibody receptor T (DSG3-CAART) cells as a potential treatment for patients with mucosal pemphigus vulgaris (mPV). Enrollment in the combination cohort of the DesCAARTes™ trial is ongoing, where patients are pre-treated with intravenous immunoglobulin (IVIg), cyclophosphamide and fludarabine prior to DSG3-CAART infusion, with the aim of improving persistence and activation of DSG3-CAART.

- **MuSK-CAART:** Cabaletta is evaluating muscle-specific kinase (MuSK) chimeric autoantibody receptor T (MuSK-CAART) cells as a potential treatment for patients with MuSK-associated myasthenia gravis (MuSK MG). Enrollment in the Phase 1, open-label MusCAARTes™ study of MuSK-CAART in patients with MuSK autoantibody-positive MG is ongoing in a cohort without preconditioning.

Fourth Quarter and Full Year 2023 Financial Results

- Research and development expenses were $17.4 million and $55.4 million for the three months ended December 31, 2023, and the full year ended December 31, 2023, respectively, compared to $12.4 million and $39.3 million for the three months ended December 31, 2022, and the full year ended December 31, 2022, respectively.

- General and administrative expenses were $5.7 million and $19.2 million for the three months ended December 31, 2023, and the full year ended December 31, 2023, respectively, compared to $3.9 million and $14.8 million for the three months ended December 31, 2022, and the full year ended December 31, 2022, respectively.

- As of December 31, 2023, Cabaletta had cash, cash equivalents and short-term investments of $241.2 million, compared to $106.5 million as of December 31, 2022.

The Company expects that its cash, cash equivalents and short-term investments as of

December 31, 2023, will enable it to fund its operating plan into the first half of 2026.

About CABA-201
CABA-201 is designed to deeply and transiently deplete CD19-positive B cells following a one-time infusion, which may enable an "immune system reset" with the potential for durable remission off therapy in patients with autoimmune diseases. To date, Cabaletta has received clearance from the FDA for four Investigational New Drug (IND) applications for CABA-201 in multiple autoimmune conditions including systemic lupus erythematosus (SLE), myositis, systemic sclerosis (SSc) and generalized myasthenia gravis (gMG). Cabaletta is conducting four Phase 1/2 RESET™ clinical trials with a total of nine cohorts that can advance simultaneously, employing a similar parallel cohort design and starting dose of 1 x 10^6 cells/kg without a dose escalation requirement.

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 encompasses two strategies: the CARTA (chimeric antigen receptor T cells for autoimmunity) strategy, with CABA-201, a 4-1BB-containing fully human CD19-CAR T, as the lead product candidate being evaluated in the RESET™ (REstoring SElf-Tolerance) clinical trials in systemic lupus erythematosus, myositis, systemic sclerosis and generalized myasthenia gravis, and the CAART (chimeric autoantibody receptor T cells) strategy, with multiple clinical-stage candidates, including DSG3-CAART for mucosal pemphigus vulgaris and MuSK-CAART for MuSK myasthenia gravis. The expanding CABA™ platform is designed to develop potentially curative therapies that offer deep and durable responses for patients with a broad range of autoimmune diseases. Cabaletta Bio's headquarters and labs are located in Philadelphia, PA.

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: Cabaletta's ability to grow its autoimmune pipeline; its ability to capitalize on and potential benefits resulting from published third-party academic clinical data; Cabaletta's future plans and strategies for its CAAR T and CARTA technologies and the company's business plans and objectives as a whole; statements regarding regulatory filings for its development programs, including the planned timing of such regulatory filings, such as IND applications, and potential review by regulatory authorities; Cabaletta's ability to retain and recognize and its expectations around the intended incentives conferred by Fast Track Designation and/or Orphan Drug Designation for CABA-201 for the treatment of multiple autoimmune diseases; Cabaletta's ability to retain and recognize and its expectations around the potential benefits and incentives provided by FDA's rare pediatric disease designation for CABA-201 and the potential benefits provided by FDA's priority review voucher; Cabaletta's expectations around the potential success and therapeutic benefits of CABA-201, including its belief that CABA-201 may enable an "immune system reset" and provide deep and durable responses in patients across an increasing number of autoimmune diseases; Cabaletta's belief that it is developing the first CD19-CAR T therapy specifically designed for patients with autoimmune disease and that it has an efficient path to initiation of registrational studies; Cabaletta's ability to realize its vision of transforming autoimmune disease treatment, including

progressing efforts to address scale in autoimmune disease, innovating to optimize the patient and physician experience and expanding the potential application of CABA-201 to multiple additional indications with well-defined patient populations; Cabaletta's belief that it is well-positioned to develop and launch the first targeted, and perhaps curative, cellular therapies for patients with autoimmune diseases; the Company's advancement of separate Phase 1/2 clinical trials of CABA-201 in patients with SLE, myositis, SSc and gMG, including updates related to status, safety data, or otherwise and the expected timing of the related data read-outs; Cabaletta's ability to accelerate its pipeline, develop meaningful therapies for patients and leverage its research and translational insights; the Company's expectations for the efficiency of the trial design for its Phase 1/2 clinical trials of CABA-201; Cabaletta's planned initial clinical data read-out in the first half of 2024 for patients with myositis and SLE treated with CABA-201; Cabaletta's planned initial clinical data read-out in the second half of 2024 for patients with SSc and gMG treated with CABA-201; use of capital, expense and other financial results in the future; ability to fund operations into the first half of 2026 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: risks related to regulatory filings and potential clearance; 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; the risk that the results observed with the similarly-designed construct employed in academic publications, including due to the dosing regimen, are not indicative of the results we seek to achieve with CABA-201; risks related to clinical trial site activation, delays in enrollment generally 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 and public health crises; 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, including in light of recent legislation; 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 December 31,		Year Ended December 31,	
	2023	**2022**	**2023**	**2022**
	Unaudited			
Operating expenses:				
Research and development	17,405	12,400	55,424	39,300
General and administrative	5,741	3,902	19,236	14,839
Total operating expenses	23,146	16,302	74,660	54,139
Loss from operations	(23,146)	(16,302)	(74,660)	(54,139)
Interest income	2,260	610	6,985	1,164
Net loss	(20,886)	(15,692)	(67,675)	(52,975)
Net loss per voting and non-voting share, basic and diluted	$ (0.46)	$ (0.52)	$ (1.65)	$ (1.81)

Selected Balance Sheet Data

	December 31,	
	2023	**2022**
	Unaudited	
Cash, cash equivalents and short-term investments	$ 241,249	$ 106,547
Total assets	253,650	116,968
Total liabilities	17,452	12,448
Total stockholders' equity	236,198	104,520

Contacts:

Anup Marda
Chief Financial Officer
investors@cabalettabio.com

William Gramig
Stern Investor Relations, Inc.
william.gramig@sternir.com

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Source: Cabaletta Bio