August 8, 2024

Cabaletta Bio Reports Second Quarter 2024 Financial Results and Provides Business Update

– Nine patients enrolled as of August 5, 2024 across theRESET™ clinical development program, including four since EULAR in June, with 22 U.S. clinical sites now enrolling–

– Additional clinical data from the RESET-Myositis™ and RESET-SLE™ trials as well as initial clinical data from the RESET-SSc™ and RESET-MG™ trials anticipated in 2H24 –

– Initial clinical and translational data from each of the first patients in the RESET-Myositis and RESET-SLE trials presented at EULAR in June 2024 –

– An LN patient with very active, refractory disease dosed with CABA-201 in late June experienced a protocol-defined dose-limiting toxicity of Grade 4 ICANS, which resolved rapidly following standard management; the independent data monitoring committee recommended the study to proceed as designed, without delay, at the current dose –

– Recently signed Lonza and Cellares agreements support progression into next stage of manufacturing strategy to support expansion of clinical supply while preparing to efficiently scale commercial supply for CABA-201 –

– Cash, cash equivalents and short-term investments total $203.2 million as of June 30, 2024, expected to support operations into the first half of 2026 –

PHILADELPHIA, Aug. 08, 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 second quarter ended June 30, 2024, and provided a business update.

“We have seen increased enrollment and additional clinical sites open since presenting positive initial clinical and translational data for the first two patients dosed with CABA-201 at the EULAR 2024 Congress in June. We look forward to sharing additional clinical data on CABA-201 in the second half of this year,” said Steven Nichtberger, M.D., Chief Executive Officer of Cabaletta. “In addition, we have recently advanced our manufacturing strategy for CABA-201 through a new CDMO agreement with Lonza and by expanding our existing fully automated manufacturing collaboration with Cellares. We have also added Sarah Yuan, Ph.D., as our Chief Technology Officer. Sarah brings substantial cell therapy development and commercial launch experience, including at bluebird bio and 2seventy bio, where she was instrumental in the regulatory approval process for Abecma™ and two other cell therapy medicines. With the momentum and milestones achieved in the second quarter and recent period, we believe we are well positioned to realize our vision of developing and launching the first curative targeted cell therapy 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 designed 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 where B cells contribute to the initiation and/or maintenance of disease.

Rheumatology Portfolio

- **Myositis (idiopathic inflammatory myopathies, IIM)**
  - In June 2024, Cabaletta reported positive initial clinical data on the first patient in the immune-mediated necrotizing myopathy (IMNM) cohort of the Phase 1/2 RESET-Myositis trial with three months of follow-up. The data were presented at a satellite symposium at the EULAR 2024 Congress.
  - Patient enrollment in the RESET-Myositis trial is ongoing and additional clinical data from the trial are expected in the second half of 2024.

- **Systemic lupus erythematosus (SLE)**
  - In June 2024, Cabaletta reported positive initial clinical data on the first patient in the SLE non-renal cohort of the Phase 1/2 RESET-SLE trial with one month of follow-up. The data were presented at a satellite symposium at the EULAR 2024 Congress.
  - In late June 2024, a lupus nephritis (LN) patient with very active, refractory disease was dosed with CABA-201 and subsequently experienced a protocol-defined dose-limiting toxicity of grade 4 immune effector cell-associated neurotoxicity syndrome (ICANS). The ICANS resolved rapidly following standard management. After data review, the Independent Data Monitoring Committee recommended that the study proceed at the current dose without delay. The Company has proposed and is implementing protocol modifications designed to improve patient safety, including enhanced monitoring for fever and neurologic symptoms along with seizure prophylaxis for all patients, in line with the practice at many academic sites including at Erlangen University, the site of the CD19-CAR T studies led by Dr. Georg Schett. Last month, the Company communicated details of the event and proposed protocol changes to all active clinical sites within the RESET clinical trial program.
  - Patient enrollment in both cohorts of the RESET-SLE trial is ongoing and additional clinical data from the trial are expected in the second half of 2024.

- **Systemic sclerosis (SSc)**
  - Patient enrollment in the Phase 1/2 RESET-SSc trial is ongoing and initial clinical data from the trial are expected in the second half of 2024.

Neurology Portfolio

- **Generalized myasthenia gravis (gMG)**
  - Patient enrollment in the Phase 1/2 RESET-MG trial is ongoing and initial clinical data from the trial are expected in the second half of 2024.

Dermatology Portfolio

- **Pemphigus vulgaris (PV)**
  - Cabaletta is working with active clinical sites to incorporate the RESET-PV™ sub-study within the Phase 1 DesCAARTes™ trial following the submission of a protocol amendment. The RESET-PV sub-study will evaluate CABA-201 as a monotherapy without preconditioning in patients with mucosal PV (mPV) and mucocutaneous PV (mcPV).

External Scientific Presentations and Publications

- In May 2024, Cabaletta's manuscript on the preclinical characterization of CABA-201 titled "Preclinical specificity and activity of a fully human 4-1BB expressing anti-CD19 CART therapy for treatment-resistant autoimmune disease" was published in *Molecular Therapy Methods & Clinical Development*. The preclinical data support the evaluation of CABA-201 for clinical development in patients with autoimmune diseases and were included within the Investigational New Drug submissions for CABA-201.

- In June 2024, Cabaletta presented positive initial clinical data from each of the first two patients dosed with CABA-201 in the RESET-Myositis and RESET-SLE trials at a EULAR European Congress of Rheumatology 2024 Industry Symposia session titled "Immune Reset: The Potential of CAR T Cell Therapy to Transform the Treatment of Patients with Autoimmune Disease" in Vienna, Austria. The initial clinical data demonstrated:
  - CABA-201 was generally well-tolerated with no serious adverse events reported for either patient through the follow-up period.
  - CABA-201 exhibited its anticipated profile of CAR T cell expansion and contraction with complete B cell depletion observed in both patients by day 15 post-infusion.
  - Improvements in both patients' specific disease measures, consistent with the academic experience of a similar 4-1BB CD19-CAR T, suggest a potential emerging clinical benefit with CABA-201.
  - Immature, naïve B cell repopulation in first IMNM patient observed at week 8 is consistent with a potential immune system reset.

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 mPV. The DesCAARTes trial is no longer dosing patients with DSG3-CAART after evaluation of clinical and translational data from the combination cohort, where patients were pre-treated with IVIg, cyclophosphamide and fludarabine prior to DSG3-CAART infusion.

- **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). Based on review of the initial clinical data and the data from the DSG3-CAART trial, the MusCAARTes™ trial is currently dosing patients in the A2 cohort, where patients are treated with MuSK-CAART without preconditioning.

Manufacturing Leadership and Strategy Updates

- In June 2024, Sarah Yuan, Ph.D., joined the Company as Chief Technology Officer. Dr. Yuan possesses over 20 years of experience in process development and manufacturing strategy leadership in the life sciences industry and most recently served as Chief Technical Operations Officer at Sigilon Therapeutics, Inc., a wholly owned subsidiary of Eli Lilly & Co. Prior to that, Dr. Yuan was Vice President of Process and Analytical Development at bluebird bio and 2seventy bio, where she was instrumental in the regulatory approval process for Abecma and two additional cell therapy medicines. Dr. Yuan reports to Gwendolyn Binder, Ph.D., President of Science and Technology of Cabaletta, and is responsible for the process and analytical development, manufacturing strategy, and supply chain operations, in addition to supporting CMC quality control.

- In July 2024, Cabaletta entered into a new manufacturing agreement with Lonza, a leading Contract Development and Manufacturing Organization (CDMO). Under the terms of the agreement, a technology transfer of the manufacturing process for CABA-201 will be performed from Cabaletta to Lonza in anticipation of being able to supply Good Manufacturing Practices (GMP) products to support any of Cabaletta's current and planned clinical trials that evaluate CABA-201, including potential late-stage clinical trials and commercial readiness activities for CABA-201.

- In August 2024, Cabaletta expanded its original November 2023 partnership with Cellares, the first Integrated Development and Manufacturing Organization (IDMO) dedicated to clinical and industrial-scale cell therapy manufacturing, following a successful initial proof-of-concept technology transfer process for the manufacture of CABA-201 using the Cell Shuttle™. The expanded partnership facilitates the potential to incorporate Cellares' manufacturing platform in the CABA-201 clinical program.

Second Quarter 2024 Financial Results

- Research and development expenses were $23.4 million for the three months ended June 30, 2024, compared to $11.8 million for the same period in 2023.

- General and administrative expenses were $6.9 million for the three months ended June 30, 2024, compared to $4.1 million for same period in 2023.

- As of June 30, 2024, Cabaletta had cash, cash equivalents and short-term investments of $203.2 million, compared to $241.2 million as of December 31, 2023.

The Company expects that its cash, cash equivalents and short-term investments as of June 30, 2024, 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 cells following a one-time infusion, which may enable an "immune system reset" with the potential for durable remission without chronic therapy in patients with autoimmune diseases. Cabaletta is evaluating CABA-201 in multiple autoimmune conditions within five disease-specific company sponsored INDs including myositis (idiopathic inflammatory myopathy, or IIM), systemic lupus erythematosus (SLE), systemic sclerosis (SSc), generalized myasthenia

gravis (gMG), and pemphigus vulgaris (PV; a sub-study to evaluate CABA-201 without preconditioning).

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 myositis, systemic lupus erythematosus, systemic sclerosis, generalized myasthenia gravis and in the RESET-PV™ sub-study within the DesCAARTes™ clinical trial in pemphigus vulgaris, along with 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-associated 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; Cabaletta's business plans and objectives as a whole; Cabaletta's ability to realize its vision of launching the first curative targeted cell therapy for patients with autoimmune diseases; Cabaletta's ability to successfully complete research and further development and commercialization of its drug candidates in current or future indications; the uncertainties inherent in clinical testing and accruing patients for clinical trials; the timing and results of Cabaletta's clinical trials, as well as its ability to conduct and complete clinical trials; expectation that clinical results will support CABA-201's safety and activity profile; statements regarding the expectations of trial modifications and prophylactic measures, continued trial operations; statements regarding the timing of regulatory filings and interactions with regulatory authorities, including such authorities' review of safety information from Cabaletta's ongoing clinical trials; Cabaletta's ability to retain and recognize and its expectations around the intended incentives conferred by Fast Track Designation for CABA-201 for the treatment of multiple autoimmune diseases; 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" with the potential for durable remission without chronic therapy in 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 and advancement of a RESET-PV sub-study within the ongoing DesCAARTes trial in PV, 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 and its expanding manufacturing partnerships; Cabaletta's ability to execute its manufacturing strategy to enable expansion of clinical supply and efficiently scale commercial supply for CABA-201; Cabaletta's planned additional clinical data read-out for patients with myositis and SLE treated with CABA-201; Cabaletta's planned initial clinical data read-outs for patients with

SSc and gMG treated with CABA-201 or otherwise; Cabaletta's ability to increase enrollment from its rapidly expanding clinical network in the RESET clinical program; Cabaletta's planned assessment of its DesCAARTes™ and MusCAARTes™ trials; 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 that modifications to trial design or approach may not have the intended benefits and that the trial design may need to be further modified; risks related to clinical trial site activation, delays in enrollment generally or enrollment rates that are lower than expected; delays related to assessment of clinical trial results; 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 or other designations 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 June 30,		Six Months Ended June 30,	
	2024	2023	2024	2023
	unaudited		unaudited	
Operating expenses:				
Research and development	$ 23,427	$ 11,797	$ 45,381	$ 24,232
General and administrative	6,852	4,093	12,929	8,614
Total operating expenses	30,279	15,890	58,310	32,846
Loss from operations	(30,279)	(15,890)	(58,310)	(32,846)
Other income:				
Interest income	2,677	1,403	5,661	2,505
Net loss	(27,602)	(14,487)	(52,649)	(30,341)
Net loss per share of voting and non-voting common stock, basic and diluted	$ (0.56)	$ (0.37)	$ (1.07)	$ (0.81)

Selected Balance Sheet Data

	June 30, 2024	December 31, 2023
	(unaudited)	
Cash, cash equivalents and investments	$ 203,225	$ 241,249
Total assets	217,418	253,650
Total liabilities	17,899	17,452
Total stockholders' equity	199,519	236,198

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Cabaletta Bio®

Source: Cabaletta Bio