Aerovate Therapeutics Announces Full-Year 2023 Financial Results and Business Highlights

March 25, 2024

- Topline data expected in June 2024 from Phase 2b portion of the IMPAHCT global Phase 2b/Phase 3 clinical trial of AV-101 for pulmonary arterial hypertension (PAH)
- Completed enrollment in the Phase 2b portion and enrolled first patient into the Phase 3 portion of the IMPAHCT trial of AV-101 in November 2023
- Expanded intellectual property portfolio with two issued patents in 2023
- Board of Directors expanded in 2023 with the addition of Donald Santel and Habib Dable, each a former CEO with PAH experience and significant product approval and launch expertise
- Cash runway into 2026, based on our current operating plan

WALTHAM, Mass., March 25, 2024 (GLOBE NEWSWIRE) -- Aerovate Therapeutics, Inc. (Nasdaq: AVTE), a clinical stage biopharmaceutical company focused on developing drugs that meaningfully improve the lives of patients with rare cardiopulmonary disease, today announced financial results for the year ended December 31, 2023, and recent business highlights.

“Aerovate reached important milestones in 2023 with the achievement of full enrollment of the Phase 2b portion and the first patient enrolled in the Phase 3 portion of our IMPAHCT trial evaluating AV-101 for patients with PAH,” said Tim Noyes, Chief Executive Officer of Aerovate. “In 2024, we look forward to presenting baseline characteristics of IMPAHCT at the ATS international conference in May, as well as announcing Phase 2b topline data in June with the full Phase 2b clinical trial results to be presented at an upcoming scientific meeting. We remain confident that delivering our proprietary formulation of imatinib directly to the pulmonary vasculature via inhalation has the potential to achieve robust efficacy with a low side effect burden in PAH patients on two or three background therapies. As such, we continue to believe that AV-101 has the potential to be an important part of the future standard of care for PAH patients.”

2023 Highlights

Enrollment Complete and Topline Data Expected in June 2024 from Phase 2b Portion of the IMPAHCT Global Phase 2b/Phase 3 Clinical Trial. IMPAHCT (Inhaled iMatinib Pulmonary Arterial Hypertension Clinical Trial) is a global Phase 2b/Phase 3 trial of AV-101, our self-administered, twice daily dry powder inhaled formulation of the antiproliferative drug imatinib for the treatment of PAH. PAH is a devastating disease impacting approximately 70,000 people in the United States and Europe. In November 2023, we announced completion of enrollment at 202 adult patients in the Phase 2b portion of the IMPAHCT trial, and we continue to expect to provide topline data in June 2024.

First Patient in Phase 3 Portion of IMPAHCT Enrolled in November 2023. Due to the seamless trial design, we were able to enroll our first patient in the Phase 3 portion of the IMPAHCT trial in the same month we completed enrollment in our Phase 2b portion of the IMPAHCT trial. Enrollment continues with over 120 clinical sites participating in the Phase 3 portion of the trial. The timing for topline Phase 3 data and the size of the Phase 3 portion of the trial will be determined based upon the results from the Phase 2b portion of the trial.

Trial Data Published and Presented at Scientific Conferences. We presented at the American Thoracic Society (ATS) 2023 International Conference results in nonclinical species that (i) direct delivery of imatinib to the lungs demonstrated increased lung exposure compared with oral or IV dosing and (ii) the type of formulation impacted lung exposure with dry powder demonstrating greater lung exposure than suspension or solution and greater lung exposure versus oral or intravenous delivery.

Expanded Intellectual Property Portfolio. We continued expanding our intellectual property portfolio in 2023 with the issuance of two additional patents bringing the total issued patents to six. In 2023, the United States Patent and Trademark Office issued Patents 11,806,349 and 11,813,263 to us, and we continue prosecuting multiple additional applications to cover our product globally.

Expanded Board of Directors with Experience in PAH. We appointed Habib Dable and Donald Santel to our Board of Directors. In March 2024, Mr. Dable was also appointed as Chair of our Board of Directors, as successor to Mark Iwicki, who remains a member of our Board. Mr. Dable is on three public biopharmaceutical company boards and was previously Chief Executive Officer of Acceleron Pharma Inc. at the time of its sale to Merck & Co., Inc. Mr. Santel serves on the board of directors of three biopharmaceutical companies and as chair of two of them. Mr. Santel previously served as Chief Executive Officer for CoTherix, Inc., a biopharmaceutical company focused on developing therapies for cardiopulmonary disease, including PAH.

2023 Financial Results

Cash, cash equivalents and short-term investments totaled $122.4 million as of December 31, 2023, compared to $135.2 million as of September 30, 2023. The decrease was primarily driven by operational costs for the three-month period ended December 31, 2023.

R&D expenses: Research and development (R&D) expenses for the year ended December 31, 2023 were $64.2 million as compared to $38.6 million for the year ended December 31, 2022. The increase in R&D expenses was due primarily to clinical trial costs, manufacturing costs, and increased

headcount-related costs in 2023 as compared to 2022.

G&A expenses: General and administrative (G&A) expenses for the year ended December 31, 2023 were $17.2 million as compared to $14.6 million for the year ended December 31, 2022. The increase in G&A expenses was due primarily to increased headcount-related costs, travel and other miscellaneous costs in 2023 as compared to 2022.

Net loss: Net loss for the year ended December 31, 2023 was $75.5 million as compared to $51.5 million for the year ended December 31, 2022. Net loss included stock-based compensation expense of $11.9 million and $5.5 million for the years ended December 31, 2023 and December 31, 2022, respectively.

Financial guidance: We expect that our cash, cash equivalents and available-for-sale investments will be sufficient to fund our operations into 2026, based on our current operating plan.

About AV-101

AV-101 is an investigational, proprietary dry powder inhaled formulation of the antiproliferative drug imatinib. Developed specifically for pulmonary arterial hypertension (PAH), AV-101 targets cellular hyperproliferation and resistance to apoptosis, driven by improper signaling in cells of the distal pulmonary arteries. By targeting the proliferation and accumulation of cells in the arteries of the lungs, we believe AV-101 has the potential to provide meaningful improvements for patients beyond the capabilities of currently approved therapies. AV-101 is designed for delivery by an easy-to-use dry powder inhaler, directly into the lungs to maximize potential clinical benefit and limit systemic adverse effects. Phase 1 results published in *ERJ Open Research* showed that AV-101 delivered by dry powder inhalation was generally well-tolerated by healthy adult volunteers with no serious adverse events reported. Aerovate has completed enrollment in the Phase 2b portion of the IMPAHCT clinical trial and is currently enrolling patients in the Phase 3 portion to evaluate the safety and efficacy of AV-101 in adults with PAH.

About the IMPAHCT Trial

IMPAHCT (Inhaled iMatinib Pulmonary Arterial Hypertension Clinical Trial) is a multi-national, placebo-controlled Phase 2b/Phase 3 trial in adults with PAH that continuously enrolled patients as the study progresses from Phase 2b to Phase 3. The Phase 2b portion of the trial will evaluate three doses of AV-101 over 24 weeks, compared to placebo, to identify an optimal dose based on the primary endpoint, change in pulmonary vascular resistance (PVR), and safety, tolerability, and other clinical measures. The Phase 3 portion of the trial will compare patients taking the optimal dose of AV-101, selected from the Phase 2b data, to placebo. The primary endpoint of the Phase 3 portion of the trial will be change in six-minute walk distance (6MWD) over 24 weeks versus placebo. More information about this trial is available at https://clinicaltrials.gov/ct2/show/NCT05036135https://clinicaltrials.gov/ct2/show/NCT05036135.

About Aerovate Therapeutics, Inc.

Aerovate is a clinical-stage biopharmaceutical company focused on developing drugs that meaningfully improve the lives of patients with rare cardiopulmonary disease. Aerovate's initial focus is on advancing AV-101, its proprietary dry powder inhaled formulation of the drug imatinib for the treatment of patients with PAH. Learn more at aerovatetx.com or follow the company on X (formerly known as Twitter) and LinkedIn.

Available Information

Aerovate announces material information to the public about the Company, its products and services, and other matters through a variety of means, including filings with the U.S. Securities and Exchange Commission (SEC), press releases, public conference calls, webcasts, the investor relations section of the Company website at ir.aerovatetx.com, and the Company's X (formerly known as Twitter) account @AerovateTx in order to achieve broad, non-exclusionary distribution of information to the public and for complying with its disclosure obligations under Regulation FD.

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. Forward-looking statements can be identified by words such as "anticipate," "believe," "continue," "could," "estimate," "expect," "future," "goal," "intend," "look forward to," "may," "plan," "potential," "predict," "project," "potential," seek," "strategy," "should," "target," "will," "would" and similar expressions regarding future periods. These forward-looking statements include, but are not limited to, statements regarding the therapeutic potential and clinical benefits of AV-101 in PAH; our anticipated timing for the release of topline data from the Phase 2b portion of the IMPAHCT trial; our expectations regarding continuing patient enrollment for the Phase 3 portion of the IMPAHCT trial; our belief that we will have capital to fund Aerovate into 2026; our expectations regarding the strength of our intellectual property portfolio globally; our business plans and objectives for AV-101, including potential regulatory submissions and approvals; the anticipated contribution of the members of our board of directors and management team to our operations and progress; and our growth and goals as a company.

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, those risks and uncertainties related to the therapeutic potential and clinical benefits of AV-101; the timing associated with patient enrollment, initiation, delivery of drug supply and continuation of our Phase 2b/Phase 3 trial of AV-101 in PAH patients; clinical trials, operations and goals; positive results from a clinical study may not necessarily be predictive of the results of future or ongoing clinical studies; regulatory developments in the United States and foreign countries; as well as those risks and uncertainties set forth more fully under the caption "Risk Factors" in our most recent Annual Report on Form 10-K filed with the SEC and subsequent filings with the SEC. 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.

Aerovate Therapeutics, Inc.
Condensed Consolidated Balance Sheets

(Unaudited)
(in thousands)

	December 31, 2023	December 31, 2022
Assets		
Cash, cash equivalents and available-for-sale securities	$ 122,439	$ 129,220
Other assets	4,979	6,081
Total assets	127,418	135,301
Liabilities and Stockholders' Equity		
Accounts payable and accrued and other current liabilities	$ 17,161	$ 7,397
Other liabilities	745	1,161
Total liabilities	17,906	8,558
Total stockholders' equity	109,512	126,743
Total liabilities and stockholders' equity	$ 127,418	$ 135,301

Aerovate Therapeutics, Inc.
Condensed Consolidated Statements of Operations and Comprehensive Loss
(Unaudited)
(in thousands, except share and per share amounts)

	Three Months Ended December 31,		Year Ended December 31,	
	2023	2022	2023	2022
Operating expenses:				
Research and development	$ 17,813	$ 12,221	$ 64,219	$ 38,622
General and administrative	4,253	3,631	17,190	14,615
Total operating expenses (1)	22,066	15,852	81,409	53,237
Loss from operations	(22,066)	(15,852)	(81,409)	(53,237)
Total other income	1,709	856	5,944	1,751
Provision for income taxes	56	25	56	25
Net loss	$ (20,413)	$ (15,021)	$ (75,521)	$ (51,511)
Net loss per share, basic and diluted	$ (0.74)	$ (0.61)	$ (2.87)	$ (2.10)
Weighted-average shares of common stock outstanding, basic and diluted	27,695,184	24,610,723	26,331,630	24,472,104

(1) Non-cash charges were $3.3 million and $11.9 million, and $1.7 million and $5.5 million for the three and twelve months ended December 31, 2023 and 2022, respectively.

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