Aerovate Therapeutics Presents Baseline Data from the Phase 2b Portion of the IMPAHCT Trial at the American Thoracic Society 2024 International Conference

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Baseline characteristics reflect a PAH population with significant disease

Topline data from Phase 2b portion of IMPAHCT study to be released in June 2024

Enrollment into the Phase 3 portion of IMPAHCT continues at more than 120 sites globally

WALTHAM, Mass., May 21, 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 presented a poster outlining baseline characteristics from all patients enrolled in the dose-ranging Phase 2b portion of the **I**nhaled i**M**atinib **P**ulmonary **A**rterial **H**ypertension **C**linical **T**rial (IMPAHCT) at the American Thoracic Society (ATS) 2024 International Conference taking place in San Diego, CA. IMPAHCT is a Phase 2b/Phase 3, randomized, double-blind, placebo-controlled, multinational trial evaluating the safety and efficacy of AV-101 in adults with pulmonary arterial hypertension (PAH).

“These baseline characteristics reflect a patient population with significant disease despite treatment with two to three targeted PAH therapeutics. This underscores the unmet need for novel therapeutics with unique mechanisms of action that address the key drivers of PAH,” said Hunter Gillies, MBChB, Chief Medical Officer of Aerovate Therapeutics. “Coupled with our operationally seamless and adaptive IMPAHCT Phase 2b/Phase 3 trial design, we believe we are well positioned to move AV-101 forward efficiently through Phase 3 development without compromising the scientific rigor required to optimize the dose of a new investigational drug for patients.”

The 202 adult patients in the Phase 2b portion of IMPAHCT are roughly split between World Health Organization functional classes II and III, with approximately 57% of patients on triple background therapy consisting of prostacyclins, endothelin receptor antagonists, and nitric oxide pathway targeting compounds. The baseline characteristics are similar to other recent studies in PAH for novel therapeutics and should support the ability to evaluate clear signals of efficacy, safety and tolerability of AV-101, a novel dry powder formulation of imatinib administered by inhalation.

“We believe that we are at the beginning stages of a new era of therapeutics in PAH that have the potential to address the root cause of disease,” said Tim Noyes, Chief Executive Officer of Aerovate Therapeutics. “Our previous Phase 1 study in healthy adult volunteers demonstrated that direct delivery of lower doses of imatinib to the lung through dry powder inhalation resulted in lower systemic exposure than achieved with 400mg of oral imatinib with no serious adverse events reported. We are looking forward to providing our Phase 2b data in June and advancing AV-101 development to provide a novel antiproliferative treatment for patients with PAH who need more options.”

Aerovate expects to present topline Phase 2b data in June and continue Phase 3 trial enrollment globally. A copy of the conference poster presentation will be available in the “Events & Presentations” section of Aerovate’s website at ir.aerovatetx.com.

About PAH
PAH is a rare, progressive orphan disease with unmet medical need that affects approximately 70,000 people in the United States and Europe. PAH can cause strain on the heart, leading to limitation of physical activity, heart failure and reduced life expectancy.

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 is designed to target 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 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/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," 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 baseline patient characteristics from the Phase 2b portion of the IMPAHCT trial and contribution of those characteristics to the evaluation of safety and efficacy measures; the clinical significance of similarities in baseline characteristics of patients in IMPAHCT as compared to prior-conducted trials, including third-party trials; our expectations regarding continuing patient enrollment for the Phase 3 portion of the IMPAHCT trial; therapeutic potential and clinical benefits of AV-101; and our anticipated timing for the release of topline data from the Phase 2b portion of the IMPAHCT trial.

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, safety and clinical benefits of AV-101; the timing associated with the identification and activation of clinical sites, patient enrollment, initiation, delivery of drug supply and continuation of our Phase 2b/Phase 3 trial of AV-101 in PAH patients; 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.

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