Milestone Pharmaceuticals Announces Resubmission of New Drug Application for Etripamil for Treatment in Paroxysmal Supraventricular Tachycardia

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MONTREAL and CHARLOTTE, N.C., March 28, 2024 (GLOBE NEWSWIRE) -- Milestone® Pharmaceuticals Inc. (Nasdaq: MIST), a biopharmaceutical company focused on the development and commercialization of innovative cardiovascular medicines, today announced the resubmission of its New Drug Application (NDA) to the U.S. Food and Drug Administration (FDA) for etripamil, the Company's lead investigational product for the management of paroxysmal supraventricular tachycardia (PSVT).

"We appreciate FDA's guidance through the resubmission process. We believe we have addressed all the issues raised in the Refusal to File letter and look forward to working with the Agency as it reviews our application," said Joseph Oliveto, President, and Chief Executive Officer of Milestone Pharmaceuticals. "If approved, we believe that etripamil nasal spray will be a valuable treatment option for patients suffering from PSVT."

The NDA for etripamil was resubmitted based on guidance from the FDA obtained in a Type A meeting, which was conducted after receipt of a Refusal to File (RTF) letter. The resubmission package included restructured data sets that captured timing of reported AEs and certain data files reformatted to facilitate FDA's analyses. No additional efficacy or safety data were requested as part of the RTF.

About Paroxysmal Supraventricular Tachycardia

An estimated two million people in the United States are currently diagnosed with PSVT which is a type of arrhythmia or abnormal heart rhythm. PSVT is characterized by episodes of sudden onset rapid heartbeats often exceeding 150 to 200 beats per minute. The heart rate spike is unpredictable and may last several hours. The rapid heart rate often causes disabling severe palpitations, shortness of breath, chest discomfort, dizziness or lightheadedness, and distress, forcing patients to limit their daily activities. The uncertainty of when an episode of PSVT will strike or how long it will persist can provoke anxiety in patients and negatively impact their day-to-day life between episodes. The impact and morbidity from an attack can be especially detrimental in patients with underlying cardiovascular or medical conditions, such as heart failure, obstructive coronary disease, or dehydration. Many health care providers are dissatisfied with the lack of effective treatment options with patients often requiring prolonged, burdensome, and costly trips to the emergency department or even invasive cardiac ablation procedures.

About Atrial Fibrillation with Rapid Ventricular Rate

An estimated five million Americans suffer from atrial fibrillation (AFib), a common arrhythmia marked by an irregular, disruptive and often rapid heartbeat. The incidence of AFib is expected to grow to approximately 10 million by 2025 and up to about 12 million by 2030. A subset of patients with AFib experience episodes of abnormally high heart rate most often accompanied by palpitations, shortness of breath, dizziness, and weakness. While these episodes, known as atrial fibrillation with rapid ventricular rate (AFib-RVR), may be treated by oral calcium channel blockers and/or beta blockers, patients frequently seek acute care in the emergency department to address symptoms. In 2016, nearly 800,000 patients were admitted to the emergency department due to AFib symptoms where treatment includes medically supervised intravenous administration of calcium channel blockers or beta blockers, or electrical cardioversion. With little available data for AFib-RVR, Milestone's initial market research indicates that 30 to 40% of patients with AFib experience one or more symptomatic episodes of RVR per year that require treatment, suggesting a target addressable market of approximately three to four million patients in 2030 for etripamil in patients with AFib-RVR.

About Etripamil

Etripamil is Milestone's lead investigational product. It is a novel calcium channel blocker nasal spray under clinical development for frequent and often highly symptomatic episodes of PSVT and AFib-RVR. It is designed as a self-administered rapid response therapy for patients thereby bypassing the need for immediate medical oversight. If approved, etripamil is intended to provide health care providers with a new treatment option to enable on-demand care and patient self-management. This portable, self-administered treatment may provide patients with active management and a greater sense of control over their condition. CARDAMYST™, the conditionally approved brand name for etripamil nasal spray, is well studied with a robust clinical trial program that includes a completed Phase 3 clinical-stage program for the treatment of PSVT and Phase 2 trial for the treatment of patients with AFib-RVR.

About Milestone Pharmaceuticals

Milestone Pharmaceuticals Inc. (Nasdaq: MIST) is a biopharmaceutical company developing and commercializing innovative cardiovascular solutions to improve the lives of people living with complex and life-altering heart conditions. The Company's focus on understanding unmet patient needs and improving the patient experience has led us to develop new treatment approaches that provide patients with an active role in self-managing their care. Milestone's lead investigational product is etripamil, a novel calcium channel blocker nasal spray that is being studied for patients to self-administer without medical supervision to treat highly symptomatic episodic attacks associated with PSVT and AFib-RVR.

Forward-Looking Statements

This press release contains forward-looking statements within the meaning of the Private Securities Litigation Reform Act of 1995. Words such as "believe," "continue," "could," "demonstrate," "designed," "develop," "estimate," "expect," "may," "pending," "plan," "potential," "progress," "will", "intend" and similar expressions (as well as other words or expressions referencing future events, conditions, or circumstances) are intended to identify forward-looking statements. These forward-looking statements are based on Milestone's expectations and assumptions as of the date of this press release. Each of these forward-looking statements involves risks and uncertainties. Actual results may differ materially from these forward-looking statements. Forward-looking statements contained in this press release include statements regarding the timing and outcomes of future interactions with the FDA; expected growth of incidence of AFib by 2030; our future target addressable market; the potential of etripamil to provide

health care providers with a new treatment option to enable on-demand care and patient self-management and provide patients with active management and a greater sense of control over their condition. Important factors that could cause actual results to differ materially from those in the forward-looking statements include, but are not limited to, whether our future interactions with the FDA will have satisfactory outcomes; whether and when, if at all, our NDA for etripamil will be approved by the FDA; whether the FDA will require additional trials or data which may significantly delay and put at risk our efforts to obtain approval and may not be successful, the risks inherent in biopharmaceutical product development and clinical trials, including the lengthy and uncertain regulatory approval process; uncertainties related to the timing of initiation, enrollment, completion, evaluation and results of our clinical trials; risks and uncertainty related to the complexity inherent in cleaning, verifying and analyzing trial data; and whether the clinical trials will validate the safety and efficacy of etripamil for PSVT or other indications, among others, general economic, political, and market conditions, including deteriorating market conditions due to investor concerns regarding inflation, Russian hostilities in Ukraine and ongoing disputes in Israel and Gaza and overall fluctuations in the financial markets in the United States and abroad, risks related to pandemics and public health emergencies, and risks related the sufficiency of Milestone's capital resources and its ability to raise additional capital in the current economic climate. These and other risks are set forth in Milestone's filings with the U.S. Securities and Exchange Commission, including in its annual report on Form 10-K for the year ended December 31, 2023, under the caption "Risk Factors," as such discussion may be updated from time to time by subsequent filings Milestone may make with the U.S. Securities & Exchange Commission. Except as required by law, Milestone assumes no obligation to update any forward-looking statements contained herein to reflect any change in expectations, even as new information becomes available.

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