Milestone Pharmaceuticals Progresses Clinical and Regulatory Activities for Etripamil in Patients with Paroxysmal Supraventricular Tachycardia

March 06, 2023

- *Both the NODE-303 and RAPID-extension studies are complete*

- *Following FDA feedback, Etripamil NDA is on-track for submission in the third quarter of 2023*

- *Potential to be the first fast-acting, patient administered treatment to break PSVT episodes if approved*

MONTREAL and CHARLOTTE, N.C., March 6, 2023 /PRNewswire/ -- Milestone Pharmaceuticals Inc. (Nasdaq: MIST), a biopharmaceutical company focused on the development and commercialization of innovative cardiovascular medicines, today announced significant progress in clinical and regulatory activities for etripamil, the Company's investigational calcium channel blocker that is administered by patients outside of the healthcare setting to treat paroxysmal supraventricular tachycardia (PSVT).

Data from the completed NODE-303 open-label safety and RAPID extension studies will be included in the etripamil PSVT New Drug Application (NDA) submission to the U.S. Food and Drug Administration (FDA) which is expected in the third quarter of 2023. The Company believes feedback received from the FDA supports and aligns with its proposed approach for the NDA.

"With the completion of the NODE-303 and RAPID-extension studies and the recent feedback from the FDA, we are confident we have the necessary data for an NDA submission in the third quarter of 2023," said Joseph Oliveto, President and Chief Executive Officer of Milestone Pharmaceuticals. "I would like to thank all those involved in the global phase 3 clinical program for the timely achievement in our quest to help patients suffering with PSVT."

"We believe that etripamil is uniquely positioned, if approved, to make a new treatment available to patients with PSVT who currently face highly symptomatic, unpredictable, and burdensome episodes of this cardiac arrhythmia. Allowing patients to take control of their condition with a self-administered, on-demand treatment will represent value – both towards patients' empowerment and potentially to the healthcare system, due to a reduced dependence on emergency department and other healthcare resources to treat PSVT," said David Bharucha, M.D., Ph.D., F.A.C.C., Chief Medical Officer of Milestone Pharmaceuticals. "We look forward to analysis of the data from the NODE-303 and RAPID-extension studies and are keenly focused on advancing our NDA submission which could result in the first and only FDA approved at-home treatment for PSVT."

In the fourth quarter of 2022, Milestone reported positive results from the Phase 3 RAPID clinical trial of etripamil in PSVT. The trial met its primary endpoint with statistical significance, with 64.3% of patients self-administering etripamil converting to sinus rhythm within 30 minutes compared to 31.2% on placebo (hazard ratio [HR] = 2.62, $p<0.001$). Additionally, safety and tolerability data from the study continue to support the potential self-use of etripamil, with findings consistent with those observed in prior trials. The most common randomized treatment emergent adverse events (RTEAEs), which are adverse events (AEs) that occurred within 24 hours of study drug administration, were related to the nasal administration site. Analyses of pooled data from the NODE-301 and RAPID trials show that etripamil treatment provided a statistically significant reduction in the use of additional medical interventions, and a statistically significant reduction in visits to the emergency department. Further to recent interactions with the FDA, the Company believes data from the RAPID trial together with the data from the already completed NODE-301 trial will fulfill the efficacy requirement for an NDA submission for etripamil in PSVT.

About Paroxysmal Supraventricular Tachycardia

Paroxysmal Supraventricular Tachycardia (PSVT) is a highly symptomatic and impactful heart arrhythmia characterized by unpredictable attacks of a racing heart that afflicts approximately two million Americans. Symptoms of PSVT, including palpitations, chest pressure, and shortness of breath are often debilitating, causing the patient to stop their current activities or avoid pursuits altogether. 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. The uncertainty of when an episode of SVT will strike or how long it will persist can provoke anxiety in patients, which can have a negative impact on their day-to-day life. Many doctors are unsatisfied by the lack of effective treatment options besides a prolonged, unpleasant, and costly trip to the emergency department or, for some patients, an invasive ablation procedure.

About Etripamil

Etripamil, Milestone's lead investigational product, is a novel calcium channel blocker nasal spray. It is designed to be a rapid-response therapy that is self-administered by the patient, without the need for direct medical oversight, for elevated and often highly symptomatic heart rate attacks associated with PSVT and AFib-RVR. If approved, etripamil is intended to provide health care providers with a new tool to enable virtual care and patient self-management, and to impart upon the patient a greater sense of control over their condition. Milestone is conducting a comprehensive development program for etripamil, with Phase 3 trials completed and an NDA soon to be submitted in the third quarter of 2023 in paroxysmal supraventricular tachycardia (PSVT). Milestone also has a Phase 2 proof-of-concept trial that is ongoing in patients with atrial fibrillation with rapid ventricular rate (AFib-RVR).

About Milestone Pharmaceuticals

Milestone Pharmaceuticals Inc. (Nasdaq: MIST) is a biopharmaceutical company focused on the development and commercialization of innovative cardiovascular medicines. Milestone's lead product candidate etripamil has completed the Phase 3 clinical program to support an NDA for the treatment of paroxysmal supraventricular tachycardia (PSVT) and is in a Phase 2 proof-of-concept trial for the treatment of patients with atrial fibrillation with rapid ventricular rate (AFib-RVR). Milestone Pharmaceuticals operates in Canada and the United States. For more information, visit www.milestonepharma.com and follow Milestone on Twitter at @MilestonePharma.

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" 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 potential of etripamil to serve as a promising therapy for PSVT patients; the potential for clinical trial data from the NODE-303 and RAPID-extension studies of etripamil nasal spray in patients with PSVT (along with results from Phase 3 RAPID clinical trial and NODE-301 trial results) to support an NDA in the third quarter of 2023 pending agency feedback; the design, progress, timing, scope, recruitment and results of the RAPID and NODE trials; Milestone's ability to execute on the remainder of the PSVT program; the receipt of milestone payments from Ji Xing, Milestone's ongoing plans to study etripamil in atrial fibrillation patients; the sufficiency of Milestone's current cash resources to support its operations, and estimates about the addressable market and commercial potential for treatments of atrial fibrillation with rapid ventricular rate. Important factors that could cause actual results to differ materially from those in the forward-looking statements include, but are not limited to, 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 and Russian hostilities in Ukraine and overall fluctuations in the financial markets in the United States and abroad, risks related to pandemics and public health emergencies, including those related to the ongoing COVID-19 pandemic, and risks related the sufficiency of Milestone's capital resources and its ability to raise additional capital. 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, 2021, under the caption "Risk Factors," as such discussion may be updated from time to time by subsequent filings we 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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