Milestone Pharmaceuticals Announces First Patient Enrolled in RAPID, a Pivotal Phase 3 Study of Etripamil in PSVT

November 18, 2020

– RAPID and completed NODE-301 studies could potentially fulfill efficacy requirement for future NDA for etripamil in patients with PSVT –

– RAPID investigates efficacy of second dose of etripamil for patients with persistent PSVT –

– RAPID results expected late 2021/early 2022 –

MONTREAL and CHARLOTTE, N.C., Nov. 18, 2020 /PRNewswire/ -- Milestone Pharmaceuticals Inc. (Nasdaq: MIST), a biopharmaceutical company focused on the development and commercialization of innovative cardiovascular medicines, today announced that the first patient has been enrolled in RAPID, a pivotal Phase 3 trial of etripamil nasal spray. Etripamil is a novel, short-acting calcium channel blocker in development for the treatment of patients with paroxysmal supraventricular tachycardia (PSVT).

"Commencement of RAPID marks an important milestone for the PSVT etripamil program," said Joseph Oliveto, President and Chief Executive Officer of Milestone Pharmaceuticals. "We remain very encouraged by physicians' reactions to the safety and efficacy results from the NODE-301 trial and believe the RAPID trial has the potential to demonstrate increased efficacy and improved overall clinical utility of etripamil. We appreciate and thank the clinical investigators, trial coordinators, etripamil trial team, and patients for their contributions and continued support of RAPID."

The RAPID trial is expected to randomize up to 500 patients and will be completed after a total of 180 confirmed supraventricular tachycardia (SVT) events are reached. Patients in the RAPID trial will be randomized 1:1 to etripamil or placebo. To maximize the potential treatment effect of etripamil, patients who do not experience symptom relief within 10 minutes of the first study drug administration will be directed to administer a second dose of study drug.

As previously announced, the primary efficacy endpoint for both the RAPID and NODE-301 trials will be time to conversion of SVT within 30 minutes following initial study drug administration, with a target p-value of less than 0.05 for each trial. Milestone expects to report data from the RAPID trial in late 2021/early 2022.

About Paroxysmal Supraventricular Tachycardia

PSVT is a rapid heart rate condition characterized by intermittent episodes of SVT that start and stop suddenly and without warning. Episodes of SVT are often associated with symptoms including palpitations, sweating, chest pressure or pain, shortness of breath, sudden onset of fatigue, lightheadedness or dizziness, fainting, and anxiety. Certain calcium channel blockers have long been approved for the treatment of PSVT as well as other cardiac conditions. However, calcium channel blockers approved for the termination of SVT episodes must be administered intravenously under medical supervision, usually in an emergency department or other acute care setting.

About Etripamil

Etripamil, the Company's lead investigational product, is designed to be a rapid-response therapy for episodic cardiovascular conditions. The novel calcium channel blocker is self-administered via a nasal spray, which may shift the current treatment paradigm for many patients with PSVT from the emergency department to the at-home setting. Milestone is conducting a comprehensive development program for etripamil, with Phase 3 trials underway in PSVT, and plans to commence a Phase 2 proof-of-concept trial in patients with atrial fibrillation with rapid ventricular rate, with subsequent studies expected in other conditions where calcium channel blockers are used.

About Milestone Pharmaceuticals

Milestone Pharmaceuticals is a biopharmaceutical company focused on the development and commercialization of etripamil, a Phase 3 clinical-stage program, for the treatment of cardiovascular indications. Milestone Pharmaceuticals operates in Canada and the United States. For more information, visit www.milestonepharma.com and follow the Company 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 "may," "will," "expect," "plan," "anticipate,", "continue" "estimate," "potential,", "prepare", "believe," "remain," "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 (i) the design, progress, timing, scope and results of the RAPID trial, (ii) potential clinical trials in other cardiac conditions and (iii) the possibility that data will support FDA approval. 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 and evaluation of clinical trials, including the RAPID trial, and whether the clinical trials will validate the safety and efficacy of etripamil for PSVT or other indications, among others, as well as risks related to pandemics and public health emergencies, including those related to COVID-19, 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 quarterly report on Form 10-Q for the quarter ended September 30, 2020, under

the caption "Risk Factors." 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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