Milestone Pharmaceuticals Announces Etripamil Phase 2 Clinical Program Success for the Treatment of Paroxysmal Supraventricular Tachycardia

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Montreal, QC, CA, May 11, 2017 – Milestone Pharmaceuticals Montreal, Canada, Inc., a clinical stage cardiovascular company, today announced positive data from its Phase 2 NODE-1 trial evaluating etripamil, a novel, potent, fast-acting and short-acting calcium channel blocker in development for the acute treatment of patients with paroxysmal supraventricular tachycardia (PSVT), a potentially debilitating cardiac arrhythmia. The data were presented in a late breaking oral presentation at Heart Rhythm 2017, the Heart Rhythm Society's 38 th Annual Scientific Sessions in Chicago. The results from the Phase 2 NODE-1 trial show that etripamil demonstrated statistically significant efficacy compared to placebo for the acute termination of PSVT induced in an electrophysiology laboratory.

The NODE-1 trial was a multi-center, placebo-controlled, double-blinded dose ranging Phase 2 trial of intranasal administration of etripamil for the conversion of induced PSVT. The primary objective of the trial was to demonstrate superiority of at least one of four intranasal etripamil doses over placebo in terminating PSVT within 15 minutes when induced in an electrophysiology laboratory.

One-hundred and four (104) patients expecting an ablation procedure were randomized into one of four etripamil treatment groups (35, 70, 105, 140mg) or placebo. After 15 minutes, PSVT conversion rates were significantly higher in the patients receiving etripamil 70mg (87%; 20/23), 105mg (75%; 15/20) and 140mg (95%; 20/21) compared to 35 percent (7/20) receiving placebo (p-values less than 0.001, 0.05, 0.001, respectively, compared to placebo). The conversion rate for patients receiving etripamil 35mg was 65% (13/20) (not significant). The median time to PSVT conversion ranged from 1.82 to 3.03 minutes across all four etripamil treatment arms.

"PSVT is an unpredictable, debilitating disorder resulting in a significant number of emergency room visits each year," said Bruce Stambler, MD, FHRS, Piedmont Heart Institute, Atlanta, GA. "A fast-acting therapy that can rapidly resolve the symptoms of PSVT would be paradigm changing, both for patients and the healthcare system. These data demonstrate the potential for etripamil to achieve this goal, and I look forward to seeing the NODE-1 results translate to a Phase 3 study in the at-home setting."

Secondary objectives of the trial were to establish a dose-related trend and evaluate the safety of etripamil. A plateauing of an efficacy response was seen starting at the 70mg dose, providing guidance for selection of the Phase 3 dose. Etripamil had an acceptable safety profile and was well tolerated. The most common adverse event was nasal discomfort and congestion. Some patients experienced a transient drop in blood pressure at the two highest doses of etripamil (105 and 140mg).

"Milestone Pharmaceuticals is committed to improving the lives of individuals with PSVT and we are highly encouraged by this Phase 2 data," said Francis Plat, MD, Chief Medical Officer, Milestone Pharmaceuticals. "The NODE-1 results are valuable in guiding further development of etripamil. We look forward to sharing the data with regulatory agencies as we finalize our Phase 3 development program. We thank all the investigators, the study site personnel and especially thank the patients for their contributions to the NODE-1 trial."

About Etripamil

Etripamil is a novel, potent, short-acting calcium channel blocker developed as a fast-acting nasal spray that can be administered by the patient to terminate paroxysmal supraventricular tachycardia (PSVT) episodes wherever and whenever they occur. A Phase 2 clinical trial (NODE-1) was successfully completed in the United States and Canada. Information regarding the NODE-1 clinical trial may be found at www.clinicaltrials.gov (study identifier NCT02296190). Milestone is actively recruiting clinical sites for a Phase 3 trial of etripamil in the at-home setting enrolling patients with confirmed diagnosis of atrioventricular nodal reentrant tachycardia (AVNRT) and atrioventricular re-entry tachycardia (AVRT). Etripamil is not approved for the treatment of PSVT or for any other indication anywhere in the world.

About Paroxysmal Supraventricular Tachycardia

Paroxysmal supraventricular tachycardia is a condition that afflicts approximately 1.7 million people and results in over 600,000 healthcare claims in the US alone per year. During a PSVT episode, patients may feel palpitations while heart rate increases dramatically and can exceed 200 beats per minute. Although the condition is not life threatening, it causes great distress to the patient and often results in a visit to a hospital emergency room where the patient is usually administered intravenous drugs and monitored until the symptoms resolve.

About Milestone Pharmaceuticals

Milestone, with headquarters in Montreal, Canada and a US subsidiary in Charlotte, NC, is a clinical stage drug development company focused on developing etripamil, a calcium channel blocker intended to provide fast-acting and short-acting treatment of PSVT episodes. For more information, please visit www.milestonepharma.com.

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