Milestone Announces Positive Phase 1 Data for MSP-2017; Supports Advancement into Phase 2 in PSVT Patients

November 24, 2014

Montreal, QC, November 24, 2014 – Milestone Pharmaceuticals, Inc. today announced data from a Phase 1 clinical trial conducted in Melbourne, Australia of MSP-2017, a novel calcium channel antagonist for the potential treatment of paroxysmal supraventricular tachycardia (PSVT). The results demonstrated that an intra-nasal formulation of MSP-2017 was well tolerated at single doses up to and including 140 mg with an excellent safety profile, desirable PK properties including rapid onset, and validating proof of concept by PR interval prolongation as measured by ECG. Milestone expects to initiate a Phase 2 clinical trial of MSP-2017 in patients with PSVT during the first half of 2015

“We believe that the pharmacological properties of MSP-2017 may result in termination of acute episodes of atrial tachycardia in patients with PSVT,” said Francis Plat, M.D., Chief Medical Officer of Milestone Pharmaceuticals. “We look forward to advancing MSP-2017 into a proof-of-concept study in patients with PSVT.”

The Phase 1 clinical trial included a randomized, double-blind, placebo-controlled, single ascending-dose study in 56 healthy volunteers. The primary objective of the study was to evaluate the safety, tolerability and pharmacokinetics of two intranasal formulations of MSP-2017 in a crossover design given as a single dose to seven successive cohorts of 8 healthy subjects each. All adverse events were mild in nature and no serious adverse events were reported.

“The results supported the selection of one of the formulations for subsequent clinical development in the Phase 2 proof-of-concept study,” said Douglas Wight, M.S., Vice-President of Drug Development. “The rapid absorption of MSP-2017 into the central compartment following nasal delivery is ideal for an immediate treatment of PSVT episodes.”

“We are pleased with the safety profile of MSP-2017 in this Phase 1 study. We did not observe QT prolongation, AV block, or significant hypotension up to the maximal feasible dose,” said Philip Sager, M.D., Chief Medical Advisor of Milestone Pharmaceuticals. “This Phase 1 study validates the product concept because a prolongation of the PR interval as measured by ECG is associated with modulation of the atrio-ventricular (AV) node in the heart, which underlies the pathology in PSVT. MSP-2017 is being developed as a self-administered episodic treatment for acute PSVT.”

The Phase 1 study was undertaken following promising results in the preclinical evaluation of MSP-2017. In a series of in vivo experiments, MSP-2017 exhibited prolongation of the PR interval as measured by ECG. These results suggest MSP-2017 can impact AV nodal conduction, and support the potential of MSP-2017 to treat PSVT episodes.

About MSP-2017

MSP-2017 is a novel and potent short-acting calcium channel antagonist for the treatment of PSVT, a potentially debilitating cardiac arrhythmia. MSP-2017 has successfully completed Phase 1 studies and is scheduled to start Phase 2 in Q1 2015. The product is being developed as a patient self-administered nasal spray to terminate PSVT episodes at-home in the unmonitored setting. MSP-2017 has a rapid onset and reaches pharmacologically relevant plasma levels within 5 minutes of administration. MSP-2017 is designed to be short-acting, allowing it to be metabolized quickly after resolution of the PSVT episode. MSP-2017 will allow patients to terminate their PSVT episodes at home and avoid ER visits. MSP-2017 is intended to address a significant unmet medical need due to the lack of convenient, rapid, safe and effective self-administered products for PSVT in the home setting.

About Milestone Pharmaceuticals

Milestone is a clinical stage drug development company developing novel small molecule therapeutics based on clinically validated mechanisms for cardiovascular diseases. Milestone’s lead product, MSP-2017, is a novel and potent short-acting calcium channel antagonist for the systemic treatment of PSVT that just completed Phase 1 studies. The Company has assembled a world-class drug development team and advisory board of key opinion leaders with significant cardiovascular expertise in cardiology, regulatory affairs, and drug development.