Milestone Pharmaceuticals Reports First Quarter 2020 Financial Results and Provides Clinical and Corporate Update

May 14, 2020

MONTREAL and CHARLOTTE, N.C., May 14, 2020 /PRNewswire/ -- Milestone Pharmaceuticals Inc. (Nasdaq: MIST), a biopharmaceutical company focused on the development and commercialization of innovative cardiovascular medicines, today reported financial results for the first quarter ended March 31, 2020 and provided a clinical and corporate update.

"While the NODE-301 trial of etripamil for patients with paroxysmal supraventricular tachycardia (PSVT) did not meet its primary endpoint over the five hour observation period, we are encouraged by the topline data from the trial. With a favorable safety and tolerability profile as well as efficacy signals observed across earlier time points, topline results from NODE-301 reinforce our belief that etripamil has the potential to serve as the first self-administered therapy for the rapid termination of supraventricular tachycardia (SVT) episodes in the at-home setting," said Joseph Oliveto, President and Chief Executive Officer of Milestone Pharmaceuticals. "We look forward to working with regulators to determine next steps, with the goal of ensuring that etripamil is able to realize its full potential in patients with PSVT. In parallel, we continue to execute on the balance of the NODE program, including NODE-301B as well as open-label safety studies NODE-302 and NODE-303."

Recent Updates

- **Reported Topline Results from Phase 3 NODE-301 Trial, Anticipates Regulatory Update in Early 3Q 2020.** In March 2020, Milestone reported topline results from its Phase 3, multicenter, randomized, double-blind, placebo-controlled NODE-301 trial of its investigational new drug, etripamil nasal spray, the Company's novel short-acting calcium channel blocker, in patients with PSVT. Despite early activity, including the conversion of 61% of etripamil patients vs. 45% of placebo patients by 45 minutes (p=0.02), a time period consistent with etripamil's known pharmacological activity, the study did not achieve its primary endpoint of time to conversion of SVT to sinus rhythm compared to placebo over the five hour period following study drug administration (p=0.12). The small number of placebo patients and prolonged efficacy measurement period was found to have confounded the results. The study did demonstrate statistically significant improvements in favor of etripamil over placebo in the important secondary endpoint of patient reported treatment satisfaction. Milestone believes the safety and tolerability data from the NODE-301 study will be supportive of at-home use of etripamil, with adverse events consistent with those observed in prior trials.

  The Company is determining next steps with regulators and expects to provide an update early in the third quarter of 2020. The Company's full PSVT clinical program, including NODE-301B, NODE-302 and NODE-303, remains ongoing. NODE-301B, which was designed to collect double-blind data from randomized patients who had not yet experienced an event after the NODE-301 trial reached its target number of adjudicated SVT events, is expected to be analyzed separately as a second safety and efficacy data set.

- **Reduction in Operating Expenses.** Milestone expects to reduce planned operating expenses by 20-25% in order to focus its efforts on an optimized clinical development pathway for etripamil that will be determined following regulatory feedback. The cuts will primarily affect pre-commercialization activities. The goal of the operating cuts is to facilitate an additional efficacy study for etripamil in PSVT and to extend the Company's cash runway. The Company will update its cash runway guidance after meeting with regulators.

- **Jeff Nelson Promoted to Chief Operating Officer.** In March 2020, Milestone announced the promotion of Jeff Nelson to Chief Operating Officer. Mr. Nelson, who joined the Company in 2018 as Vice President of Program Management, brings to this new role over 15 years of experience in the pharmaceutical and biotech field, working primarily in project management, clinical operations, regulatory affairs, drug supply and distribution and public finance.

First Quarter 2020 Financial Results

- As of March 31, 2020, Milestone had cash and cash equivalents of $102 million and 24.6 million shares outstanding.
- Research and development expense for the first quarter of 2020 was $11.9 million compared with $7.8 million for the prior year period. The increase in expense was primarily driven by increased clinical development costs and manufacturing and formulation activities supporting its Phase 3 clinical trials.
- General and administrative expenses for the first quarter of 2020 were $2.7 million compared with $1.0 million for the prior year period. The increase was driven by additional headcount, professional fees and increased insurance costs.
- Commercial expense for the first quarter of 2020 was $2.2 million and remained consistant with the prior year period.
- For the first quarter of 2020, operating loss was $16.8 million compared to $10.9 million for the prior year period.

About Paroxysmal Supraventricular Tachycardia

Paroxysmal supraventricular tachycardia (PSVT) is a rapid heart rate condition characterized by intermittent episodes of supraventricular tachycardia (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, when calcium channel blockers are used for the termination of SVT episodes, they 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 atrial fibrillation patients 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 innovative cardiovascular medicines. 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," "estimate," "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 clinical trials, (ii) potential interactions with regulators, (iii) future operating expense reductions and (iv) the possibility that data will support future development. 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, 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 our capital resources and our 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 March 31, 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.

INTERIM CONDENSED CONSOLIDATED STATEMENTS OF LOSS AND COMPREHENSIVE LOSS
(Unaudited)

[In US dollars]	'000 Three months ended March 31, **2020** $	 **2019** $
Operating expenses		
Research and development, net of tax credits	**11,872**	7,765
General and administrative	**2,703**	979
Commercial	**2,183**	2,186
Loss from operations	**(16,758)**	(10,930)
Interest income, net of bank charges	**415**	500
Loss and comprehensive loss before income taxes	**(16,343)**	(10,430)
Income tax expense	**—**	22
Net loss and comprehensive loss for the period	**(16,343)**	(10,452)
Weighted average number of shares outstanding, basic and diluted	**24,548,777**	603,040
Net loss per share, basic and diluted	**$ (0.67)**	$ (17.32)

INTERIM CONDENSED CONSOLIDATED BALANCE SHEETS
(Unaudited)

[In US dollars]	'000	
	March 31, 2020	December 31, **2019**
	$	$
ASSETS		
Current Assets		
Cash and Cash Equivalents	**101,816**	119,818
Prepaid expenses and other current assets	**2,508**	2,681
Total current assets	**104,324**	122,499
Operating lease right of use asset	**451**	524
Property and equipment	**381**	405
Total assets	**105,156**	123,428
LIABILITIES		
Current liabilities		
Accounts payable and accrued liabilities	**5,170**	7,997
Operating lease liabilities	**196**	330
Total current liabilities	**5,366**	8,327
Operating lease liabilities	**158**	184
Total liabilities	**5,524**	8,511
Shareholders' Equity		
Share capital		
Common shares, no par value, unlimited shares authorized, 24,559,470 shares issued at March 31, 2020 and 24,505,748 shares issued at December 31, 2019	**226,378**	226,245
Additional paid in Capital	**4,730**	3,805
Cumulative translation adjustment	**(1,634)**	(1,634)
Accumulated Deficit	**(129,842)**	(113,499)
Total shareholders' equity	**99,632**	114,917
Total liabilities and shareholders' deficit	**105,156**	123,428

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