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

November 13, 2020

MONTREAL and CHARLOTTE, N.C., Nov. 13, 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 third quarter ended September 30, 2020 and provided a clinical and corporate update.

"We remain on track to reopen enrollment in the pivotal Phase 3 RAPID trial by year-end, and are confident that the updated study design will help us to best characterize the potential clinical utility of our product candidate etripamil in patients with paroxysmal supraventricular tachycardia (PSVT)," said Joseph Oliveto, President and Chief Executive Officer of Milestone Pharmaceuticals. "In light of the safety and efficacy data from the completed NODE-301 trial, as well as positive feedback from the physicians charged with treating patients with PSVT, we believe that etripamil has the potential to serve as a much needed at-home intervention for this population. Backed by a strong balance sheet, which includes gross proceeds of $51.7 million from our recent public offering, we look forward to continuing to execute the etripamil PSVT program, with the goal of bringing this investigational therapy to as many appropriate patients as possible if approved by the FDA."

Recent Updates

- **Pivotal Phase 3 RAPID Trial on Track to Reopen by Year-End.** The Company remains on track to reopen enrollment in the pivotal Phase 3 RAPID trial by the end of the year. 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 help 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 trial and the already-completed NODE-301 trial 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. The RAPID and completed NODE-301 trials could potentially serve to fulfill the efficacy requirement for a future New Drug Application (NDA) for etripamil in patients with PSVT.
- **Raised $51.7 Million in Public Offering.** In October 2020, Milestone announced the closing of an underwritten public offering of 5,095,897 of its common shares and, to certain investors in lieu thereof, pre-funded warrants to purchase 4,761,903 of its common shares at an exercise price of $0.01 per share. The public offering price of each common share was $5.25 and the public offering price of each pre-funded warrant was $5.24 per underlying share. Total gross proceeds to Milestone were approximately $51.7 million before deducting underwriting discounts and offering expenses.
- **Recent Changes to Board of Directors.** In September 2020, Milestone announced the appointment of highly accomplished industry veterans Lisa Giles and Robert Wills to its Board of Directors. In addition, the Company today announced that Dr. Wills will replace Paul R. Edick as Chairman of the Board of Directors. Mr. Edick will be stepping down from the Board, effective January 1, 2021, to focus on his role as Chief Executive Officer at Xeris Pharmaceuticals and their recent product launch.

Mr. Oliveto added, "On behalf of the entire Board of Directors, I would like to thank Paul for his insights and contributions over the last two years. We wish him the very best of luck in all of his future endeavors."

Third Quarter 2020 Financial Results

- As of September 30, 2020, Milestone had cash, cash equivalents, and short-term investments of $102.9 million, 24.7 million shares outstanding and 6.7 million pre-funded warrants outstanding.
- Research and development expenses for the third quarter of 2020 were $8.2 million compared with $9.5 million for the prior year period. For the nine months ended September 30, 2020, research and development expenses were $28.7 million compared with $27.8 million for the prior year period. The increase for the nine-month period ending September 30, 2020 reflects increased clinical development costs supporting Milestone's Phase 3 clinical trials and efforts in developing a clinical trial pathway for etripamil.
- General and administrative expenses for the third quarter of 2020 were $3.0 million compared with $2.1 million for the prior year period. For the nine months ended September 30, 2020, general and administrative expenses were $8.6 million compared with $4.7 million for the prior year period. The increase in the nine-month period ending September 30, 2020 reflects increasing insurance costs, as well as additional professional fees and head count to support the compliance requirements of being a public company.
- Commercial expenses for the third quarter of 2020 were $0.9 million compared with $2.1 million for the prior year period.

For the nine months ended September 30, 2020, commercial expense was $4.6 million compared with $6.4 million for the prior year period. The decrease in expenses in the nine-month period ending September 30, 2020 reflects efforts in reducing operating expenses affecting primarily pre-commercialization activities as Milestone focused its efforts on an optimized clinical development pathway for etripamil.

- For the third quarter of 2020, operating loss was $12.1 million compared to $13.7 million in 2019. For the nine months ended September 30, 2020, Milestone's operating loss was $41.9 million compared to $39.0 million in the prior year period.

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 has the potential to 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.

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," "estimate," "potential," "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 the potential of etripamil as a promising therapy for PSVT patients, the design, progress, timing, scope and results of the RAPID trial , Milestone's expectations regarding an NDA filing and potential FDA approval for etripamil, Milestone's ability to execute on the remainder of the PSVT program and Milestone's plans to study etripamil in atrial fibrillation patients. 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 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.

INTERIM CONDENSED CONSOLIDATED STATEMENTS OF LOSS AND COMPRENHENSIVE LOSS

(Unaudited)

[In US dollars]	**'000** Three months ended September 30,		**'000** Nine months ended September 30,	
	2020	2019	**2020**	2019
	$	$	$	$
Operating expenses				
Research and development, net of tax credits	**8,228**	9,545	**28,722**	27,836
General and administrative	**2,952**	2,104	**8,611**	4,725
Commercial	**905**	2,076	**4,615**	6,428
Loss from operations	**(12,085)**	(13,725)	**(41,948)**	(38,989)
Interest income, net of bank charges	**89**	821	**630**	1,993
Loss and comprehensive loss before income taxes	**(11,996)**	(12,904)	**(41,318)**	(36,996)
Income tax recovery	**(17)**	(73)	**(17)**	(55)
Net loss and comprehensive loss for the period	**(11,979)**	(12,831)	**(41,301)**	(36,941)
Weighted average number of shares outstanding, basic and diluted	**29,774,065**	24,490,742	**26,329,581**	12,848,974
Net loss per share, basic and diluted	$ (0.40)	$ (0.52)	$ (1.57)	$ (2.87)

INTERIM CONDENSED CONSOLIDATED BALANCE SHEETS
(Unaudited)

[In US dollars]	'000	
	September 30, 2020	December 31, 2019
	$	$
ASSETS		
Current Assets		
Cash and Cash Equivalents	**102,910**	119,818
Prepaid expenses and other current assets	**4,509**	2,681
Total current assets	**107,419**	122,499
Operating lease right of use asset	**1,045**	524
Property and equipment	**333**	405
Total assets	**108,797**	123,428
LIABILITIES		
Current liabilities		
Accounts payable and accrued liabilities	**5,647**	7,997
Operating lease liabilities	**234**	330
Total current liabilities	**5,881**	8,327
Operating lease liabilities	**718**	184
Total liabilities	**6,599**	8,511
Shareholders' Equity		
Share capital		
Common shares, no par value, unlimited shares authorized, 24,727,000 shares issued at September 30, 2020 and 24,505,748 shares issued at December 31, 2019	**226,758**	226,245
Pre-funded Warrants - 6,655,131 issued at September 30, 2020	**24,770**	-
Additional paid in Capital	**7,104**	3,805
Cumulative translation adjustment	**(1,634)**	(1,634)
Accumulated Deficit	**(154,800)**	(113,499)
Total shareholders' equity	**102,198**	114,917
Total liabilities and shareholders' deficit	**108,797**	123,428

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