



Milestone
PHARMACEUTICALS

Milestone Pharmaceuticals Reports Fourth Quarter and Year-end 2019 Financial Results and Provides Clinical and Corporate Update

March 6, 2020

– Topline data from Phase 3 NODE-301 trial of etripamil in patients with PSVT expected this month –

MONTREAL and CHARLOTTE, N.C., March 6, 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 fourth quarter and year ended December 31, 2019 and provided a clinical and corporate update.

"Our team is keenly focused on delivering the near-term topline readout of the pivotal Phase 3 efficacy and safety trial, NODE-301," said Joseph Oliveto, President and Chief Executive Officer of Milestone Pharmaceuticals. "Results from NODE-301, if favorable, have the potential to serve as the sole efficacy registration trial required for an NDA submission with the United States (U.S.) Food and Drug Administration (FDA), bringing us one step closer to providing paroxysmal supraventricular tachycardia (PSVT) patients with a much-needed novel therapeutic option. Supported by compelling results from the Phase 2 NODE-1 trial, we believe etripamil has the potential to alter the PSVT treatment paradigm as the first self-administered therapy for the rapid termination of supraventricular tachycardia (SVT) episodes wherever and whenever they occur."

Mr. Oliveto added, "As we focus on topline results from NODE-301, we remain diligent in executing on the balance of our Phase 3 program of etripamil for PSVT, including the NODE-302 and NODE-303 safety studies, preparing for the potential commercialization of etripamil, and building out our pipeline beyond PSVT. This includes the expected initiation of our first clinical trial of etripamil in patients with atrial fibrillation and rapid ventricular rate."

Recent Updates

- **Topline Data from NODE-301 Trial Expected This Month.** Milestone expects to report topline data for the NODE-301 trial this month. The NODE-301 trial is a Phase 3, multicenter, randomized, double-blind, placebo-controlled trial of etripamil, the Company's novel short-acting calcium channel blocker, designed to terminate SVT episodes in the at-home setting. The primary endpoint of the NODE-301 trial is time to conversion of PSVT to sinus rhythm after the administration of trial drug, as confirmed by a central independent adjudication committee. A statistically significant result for NODE-301 would support the trial's ability to fulfill the U.S. FDA's previously guided efficacy review requirement for etripamil.

Milestone's pivotal Phase 3 program of etripamil in PSVT, which was designed in consultation with U.S. and European Union regulatory authorities, consists of three distinct trials: NODE-301, the sole efficacy trial; NODE-302, the ongoing open-label safety extension trial; and NODE-303, the ongoing global safety trial and the largest trial ever conducted in PSVT. Additional blinded data will be collected from randomized patients who have not yet experienced an event at the time the NODE-301 trial reaches its target number of adjudicated SVT events. These data will be analyzed separately as a secondary data set, referred to as NODE-301B, and may contribute further to sub-population analyses and pharmacoeconomic assessments of the NODE-301 trial.

- **Enrolled First Patient in NODE-303 Trial.** In October 2019, Milestone announced enrollment of the first patient in the Company's Phase 3 open-label, global safety trial of etripamil in patients with PSVT. The trial will primarily evaluate the safety of etripamil when self-administered without medical supervision during single or multiple SVT episodes. Important secondary measures include efficacy, patient quality of life and pharmacoeconomic assessments. The trial will enroll up to 3,000 patients in order to collect data on approximately 1,000 patients who did not participate in NODE-301 or its open-label safety extension trial, NODE-302.
- **Jeff Nelson Promoted to Chief Operating Officer.** Milestone today announced the recent 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.
- **Richard C. Pasternak, M.D. Appointed to Board of Directors.** In November 2019, Milestone announced the appointment of Richard C. Pasternak, M.D. to its Board of Directors. Dr. Pasternak brings to Milestone over 40 years of clinical, academic, and biopharmaceutical industry experience in the area of cardiology.

Fourth Quarter 2019 Financial Results

- As of December 31, 2019, Milestone had cash, cash equivalents, and short-term investments of \$119.8 million compared

to \$86.0 million as of December 31, 2018, and 24.5 million shares outstanding.

- Research and development expense for the fourth quarter of 2019 was \$14.1 million compared with \$7.3 million for the prior year period. For the full year ended December 31, 2019, research and development expense was \$42.0 million compared with \$16.8 million for the prior year.
- General and administrative expense for the fourth quarter of 2019 was \$2.3 million compared with \$1.2 million for the prior year period. For the full year ended December 31, 2019, general and administrative expense was \$7.0 million compared with \$3.1 million for the prior year.
- Commercial expense for the fourth quarter of 2019 was \$2.5 million compared with \$1.6 million for the prior year period. For the full year ended December 31, 2019, commercial expense was \$8.9 million compared with \$3.9 million for the prior year.
- For the fourth quarter of 2019, operating loss was \$18.9 million compared to \$10.1 million for the prior year period. For the full year ended December 31, 2019, Milestone's operating loss was \$57.9 million compared to \$23.8 million for the prior year.

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 utilized.

About Milestone Pharmaceuticals

Milestone 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) the anticipated timing of disclosure of results of clinical trials, (iii) the potential benefits and success of the commercialization of product candidates, and (iv) the likelihood 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 and completion of clinical trials, and whether the clinical trials will validate the safety and efficacy of etripamil for PSVT or other indications, among others. These and other risks are set forth in Milestone's filings with the U.S. Securities and Exchange Commission, including in its annual report on Form 10-K for the year ended December 31, 2019, 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.

CONSOLIDATED STATEMENTS OF LOSS AND COMPREHENSIVE LOSS

[In US dollars]	'000		'000	
	Three months ended December 31,		Twelve months ended December 31,	
	2019	2018	2019	2018
	\$	\$	\$	\$
Operating expenses				
Research and development, net of tax credits	14,149	7,296	41,985	16,849
General and administrative	2,279	1,218	7,004	3,052
Commercial	2,464	1,592	8,892	3,921
Loss from operations	(18,892)	(10,106)	(57,881)	(23,822)
Interest income, net of bank charges	604	446	2,596	711

Loss and comprehensive loss before income taxes	(18,288)	(9,660)	(55,285)	(23,111)
Income tax (recovery) expense	-	56	(56)	74
Net loss and comprehensive loss for the year	(18,288)	(9,716)	(55,229)	(23,185)
Weighted average number of shares outstanding, basic and diluted	24,496,347	441,601	15,784,750	319,202
Net loss per share, basic and diluted	\$ (0.75)	\$ (22.00)	\$ (3.50)	\$ (72.63)

CONSOLIDATED BALANCE SHEET

[In US dollars]

	'000	
	December 31, 2019	December 31, 2018
	\$	\$
ASSETS		
Current Assets		
Cash, Cash Equivalents and Short-term Investments	119,818	85,976
Prepaid expenses and other current assets	2,681	2,075
Total current assets	122,499	88,051
Operating lease right of use asset	524	-
Property and equipment	405	30
Total assets	123,428	88,081
LIABILITIES		
Current liabilities		
Accounts payable and accrued liabilities	7,997	4,477
Operating lease liabilities	330	-
Income taxes payable	-	56
Total current liabilities	8,327	4,533
Operating lease liabilities	184	-
Total liabilities	8,511	4,533
Convertible preferred shares	-	138,758
Shareholders' Equity (deficit)		
Share capital		
Common shares, no par value, unlimited shares authorized, 24,505,748 shares issued at December 31, 2019 and 596,787 shares issued at December 31, 2018		
	226,245	2,039
Additional paid-in capital	3,805	2,655
Cumulative translation adjustment	(1,634)	(1,634)
Accumulated deficit	(113,499)	(58,270)
Total shareholders' equity (deficit)	114,917	(55,210)
Total liabilities and shareholders' equity (deficit)	123,428	88,081

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