



Milestone[™]
PHARMACEUTICALS

Milestone Pharmaceuticals Reports Third Quarter 2019 Financial Results and Provides Clinical and Corporate Update

November 13, 2019

– NODE-301 topline data readout expected in mid-1H20 –

– Richard Pasternak, M.D. appointed to the Company's Board of Directors –

MONTREAL and CHARLOTTE, N.C., Nov. 13, 2019 /PRNewswire/ -- Milestone Pharmaceuticals Inc. (Nasdaq: MIST), a Phase 3 clinical-stage biopharmaceutical company dedicated to developing and commercializing etripamil for the treatment of cardiovascular indications, today reported financial results for the third quarter ended September 30, 2019 and provided a clinical and corporate update.

"Thanks to the hard work of our study team, a dedicated group of clinical sites, and, importantly, the patients participating in our study, NODE-301 continues to exceed our enrollment and PSVT event rate expectations, with topline data now expected in the middle of the first half of 2020," said Joseph Oliveto, President and Chief Executive Officer of Milestone Pharmaceuticals. "In addition, progress continues with efforts to ramp up our recently initiated global safety study, NODE-303, the largest study ever conducted in PSVT."

Mr. Oliveto added, "I am honored to welcome Dr. Richard Pasternak to our Board of Directors. A cardiologist by training, he brings a wealth of cardiovascular drug development experience that incorporates academia, large pharma and small entrepreneurial companies."

NODE-301 Update

Milestone today announced that it now expects to report topline data for the NODE-301 trial in the middle of the first half of 2020. 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, for terminating paroxysmal supraventricular tachycardia (PSVT) episodes in the outpatient setting. The study is designed to treat an enriched population of those PSVT patients who historically experience 20 minutes or longer PSVT episodes or episodes requiring termination in the emergency department.

The primary endpoint of the NODE-301 study is time to conversion of PSVT to sinus rhythm after the administration of study drug, as confirmed by a central independent adjudication committee. Secondary study endpoints include relief of symptoms commonly associated with an episode of PSVT such as heart palpitations, chest pain, anxiety, shortness of breath, dizziness, or fainting, and rating of treatment satisfaction questionnaire for medication (TSQM).

As previously announced, after the NODE-301 trial reaches its target number of adjudicated PSVT events, collection of blinded data from randomized patients who have not yet experienced an event will continue. 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.

Recent Updates

- **Richard C. Pasternak, M.D. Appointed to Board of Directors.** The Company also today announced that Richard C. Pasternak, M.D. has been appointed to its Board of Directors. He brings to Milestone over 40 years of clinical, academic, and biopharmaceutical industry experience in the area of cardiology. He will serve as a member of the Company's compensation committee.
Dr. Pasternak recently retired from Cerenis Therapeutics (now ABIONYX Pharma), a French publicly-traded company focused on developing treatments for cardiovascular diseases, where he served as Chief Executive Officer and Chair of the Board of Directors. He previously served as Vice President, Head of Cardiovascular Clinical Research, and Head of Global Scientific Affairs and Scientific Leadership, at Merck & Co. from 2004 to 2010. Prior to joining Merck, he was the Director of Preventive Cardiology and Cardiac Rehabilitation at Massachusetts General Hospital, and an Associate Professor of Medicine at Harvard Medical School.
Dr. Pasternak is currently a Clinical Professor at the Weill Cornell Medical College, and serves on the Boards of Anthos Therapeutics and Magenta Medical Ltd. He previously served on the Boards of Essentialis Therapeutics and Haptocure Ltd., as well as several nonprofit organizations. He was also previously a senior advisor to Bay City Capital and Bridge Medicines. Dr. Pasternak has authored more than 100 publications and has lectured internationally on cardiovascular disease drug development. He received his B.A. and M.D. from Yale University, and completed his medical and cardiology training at Massachusetts General Hospital.
- **Company to Commence Clinical Evaluation of Etripamil in Atrial Fibrillation with Rapid Ventricular Rate (RVR).** In 2020, Milestone plans to initiate a proof-of-concept clinical trial of etripamil for the treatment of patients with atrial fibrillation with RVR, another type of supraventricular tachycardia in which most patients experience episodes of elevated heart rates and for which L-type calcium channel blockers are approved for rate control.
- **Enrolled First Patient in NODE-303 Study.** In October 2019, Milestone announced enrollment of the first patient in the Company's Phase 3 open-label, global safety study of etripamil in patients with PSVT. The study will primarily evaluate the safety of etripamil when self-administered without medical supervision during single or multiple PSVT episodes. Important secondary measures include efficacy, patient quality of life and pharmacoeconomic assessments. The study represents the

largest study ever conducted in PSVT, assessing up to 1,500 patient episodes from patients who did not participate in NODE-301 or its open-label safety extension study, NODE-302.

- **Announced Appointment of Amit Hasija as Chief Financial Officer and Executive Vice President of Corporate Development.** In September 2019, the Company announced the appointment of Amit Hasija as Chief Financial Officer and Executive Vice President of Corporate Development. Mr. Hasija brings to Milestone two decades of experience in corporate finance and business development within the healthcare industry.

Third Quarter 2019 Financial Results

- As of September 30, 2019, Milestone had cash, cash equivalents, and short-term investments of \$136.5 million and 24.5 million shares outstanding.
- Research and development expense for the third quarter of 2019 was \$9.5 million compared with \$3.9 million for the prior year period. For the nine months ended September 30, 2019, research and development expense was \$27.8 million compared with \$9.6 million for the prior year period. The increase in 2019 amounts reflects spending on Milestone's full Phase 3 clinical program evaluating etripamil for the treatment of PSVT.
- General and administrative expenses for the third quarter of 2019 were \$2.1 million compared with \$0.6 million for the prior year period. For the nine months ended September 30, 2019, general and administrative expense was \$4.7 million compared with \$1.8 million for the prior year period. During 2019, Milestone increased its managerial headcount and, as a result, the related personnel costs. In addition, Milestone incurred increased spending for consulting fees, recruiting fees and professional fees, including legal and accounting services incurred to support its IPO.
- Commercial expense for the third quarter of 2019 was \$2.1 million compared with \$1.2 million for the prior year period. For the nine months ended September 30, 2019, commercial expense was \$6.4 million compared with \$2.3 million for the prior year period. These increases reflect increased commercial headcount and related costs, continued commercial and market research, increases in Milestone's patient advocacy activities and costs for its medical affairs team focused on key opinion leaders' engagement and disease awareness.
- For the third quarter of 2019, operating loss was \$12.9 million compared to \$5.7 million in 2018. For the nine months ended September 30, 2019, Milestone's operating loss was \$37.0 million compared to \$13.5 million in the prior year period.

About Etripamil in Paroxysmal Supraventricular Tachycardia

Paroxysmal Supraventricular Tachycardia (PSVT) is a rapid heart rate condition that starts and stops without warning, often experienced by patients with symptoms including palpitations, sweating, chest pressure or pain, shortness of breath, sudden onset of fatigue, lightheadedness or dizziness, fainting and anxiety. Calcium channel blockers have long been approved for the treatment of PSVT as well as other cardiac conditions, however, for episodes of PSVT calcium channel blockers are currently administered intravenously under medical supervision, usually in the emergency department. By contrast, etripamil is designed to serve as a self-administered therapy for the rapid termination of episodes of PSVT. With its combination of convenient delivery, rapid onset and short duration of action, etripamil has the potential to shift the current treatment paradigm for PSVT away from the burdensome and costly emergency department settings by treating episodes of PSVT wherever and whenever they occur.

About Milestone Pharmaceuticals

Milestone is a Phase 3 clinical-stage biopharmaceutical company dedicated to developing and commercializing the investigational new drug etripamil for the treatment of cardiovascular indications. Milestone is actively recruiting patients for a Phase 3 clinical trial of etripamil for the treatment of PSVT. Milestone plans to initiate a Phase 2 clinical trial in atrial fibrillation, another rapid heart rate condition, and expects to subsequently initiate an additional Phase 2 clinical trial in angina to establish proof-of-concept for the broader use of etripamil.

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 quarterly report on Form 10-Q for the period ended September 30, 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.

CONDENSED CONSOLIDATED STATEMENTS OF LOSS AND COMPREHENSIVE LOSS

(Unaudited)

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'000

[In US dollars]

	Three months ended September 30,		Nine months ended September 30,	
	2019	2018	2019	2018
Operating expenses				
Research and development, net of tax credits	\$ 9,545	\$ 3,911	\$ 27,836	\$ 9,552
General and administrative	2,104	646	4,725	1,834
Commercial	2,076	1,228	6,428	2,329
Loss from operations	\$ (13,725)	\$ (5,785)	\$ (38,989)	\$ (13,715)
Interest income, net of bank charges	821	85	1,993	265
Loss and comprehensive loss before income taxes	(12,904)	(5,700)	(36,996)	(13,450)
Income tax (recovery) expense	(73)	—	(55)	18
Net loss and comprehensive loss for the year	\$ (12,831)	\$ (5,700)	\$ (36,941)	\$ (13,468)
Weighted average number of shares outstanding, basic and diluted	24,490,742	282,771	12,848,974	277,917
Net loss per share, basic and diluted	\$ (0.52)	\$ (20.16)	\$ (2.87)	\$ (48.46)

CONDENSED CONSOLIDATED BALANCE SHEETS

(Unaudited)

[In US dollars]


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	September 30, 2019	December 31, 2018
ASSETS		
Current Assets		
Cash, Cash Equivalents and Short-term Investments	\$ 136,501	\$ 85,976
Prepaid expenses and other current assets	4,470	2,075
Total current assets	140,971	88,051
Operating lease right of use asset	593	—
Property and equipment	328	30
Total assets	\$ 141,892	\$ 88,081
LIABILITIES		
Current liabilities		
Accounts payable and accrued liabilities	\$ 8,478	\$ 4,477
Operating lease liabilities	358	—
Income taxes payable	—	56
Total current liabilities	8,836	4,533
Operating lease liabilities	224	-
Total liabilities	9,060	4,533
Convertible preferred shares	-	138,758
Shareholders' Equity (Deficit)		
Share capital		
Common shares, no par value, unlimited shares authorized, 24,490,742 shares issued at September 30, 2019 and 596,787 shares issued at December 31, 2018	226,211	2,039
Additional paid in Capital	3,466	2,655
Cumulative translation adjustment	(1,634)	(1,634)
Deficit	(95,211)	(58,270)
Total shareholders' equity (deficit)	132,832	(55,210)
Total liabilities, convertible preferred shares and shareholders' equity (deficit)	\$ 141,892	\$ 88,081

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