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Milestone Pharmaceuticals Provides Positive Regulatory Updates for Etripamil Pivotal Program in PSVT and Reports Second Quarter 2019 Financial Results

August 13, 2019

- FDA agrees to allow initiation of NODE-303 open-label safety study without test dose and in population consistent with ongoing studies –
- NODE-301 trial upsized from 100 to 150 adjudicated PSVT events to satisfy EMA requirements; Company remains on track for topline readout in 1H20 –
- Company to host mid-year business update conference call today at 8:00 a.m. ET -

MONTREAL and CHARLOTTE, N.C., Aug. 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 announced positive regulatory updates based on its interaction with the U.S. Food and Drug Administration (FDA) that advance its pivotal program of etripamil, the Company's novel, potent and short-acting calcium channel blocker, in patients with paroxysmal supraventricular tachycardia (PSVT). The Company also reported financial results for the second quarter ended June 30, 2019.

"We are very encouraged by our most recent interaction with the FDA and remain well positioned for continued execution of our etripamil pivotal program in PSVT," said Joseph Oliveto, President and Chief Executive Officer of Milestone Pharmaceuticals. "Following this interaction, we now look forward to beginning enrollment in our NODE-303 open-label global safety study before the end of the year. Execution of our pivotal Phase 3 NODE-301 trial is also tracking ahead of our initial projections, and upsizing of the trial will enable us to satisfy a European Medicines Agency (EMA) regulatory request, increase the study's power and potentially strengthen valuable sub-population analyses and pharmacoeconomic assessments, all while remaining on schedule to report topline data in the first half of 2020. Etripamil has the potential to serve as the first-ever self-administered therapy for the rapid termination of episodes of PSVT wherever and whenever they occur, and our continued progress takes us steps closer to realizing this potential to help patients living with PSVT." Nouvelles de nature réglementaire

Regulatory Updates

Following interaction with the FDA, the Company announced several regulatory updates to its etripamil pivotal program in patients with PSVT:

- **Initiation of NODE-303 open-label safety study.** Based on a review of etripamil safety data to date, the Company proposed and the FDA has agreed to allow initiation of patient enrollment in the NODE-303 study in a population consistent with ongoing studies. This includes older patients and those patients taking concomitant beta-blockers and calcium channel blockers, and without the in-office safety test dose that is currently required in the NODE-301 study. NODE-303 is an open-label global safety trial that will assess up to 1,500 patient events from patients who did not participate in NODE-301 or its open-label extension study, NODE-302. NODE-303 will evaluate the safety of etripamil when self-administered without medical supervision and evaluate the safety and efficacy of etripamil during single or multiple PSVT episodes. Milestone expects to begin enrolling patients in the study before the end of the year.



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- **NODE-301 Study Upsized to Include 150 Adjudicated PSVT Events.** Milestone has received agreement from the FDA to increase the target number of PSVT events in the Phase 3 NODE-301 trial to 150 events, up from 100 events. The upsized trial, which satisfies a regulatory request from the EMA to eliminate un-blinded, third-party data reviews for purposes of handling potential randomization imbalances, increases the study's power and potentially strengthens valuable sub-population analyses and pharmacoeconomic assessments. The rate of events in NODE-301, which remains blinded, is tracking ahead of the Company's initial projections, enabling Milestone to maintain the expectation of topline data in the first half of 2020, even with the additional PSVT events. Further, after the NODE-301 trial reaches its target 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.

The NODE-301 trial is a Phase 3, multicenter, randomized, double-blind, placebo-controlled trial of etripamil versus placebo for terminating PSVT episodes in the outpatient setting. 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 discussed with the FDA, NODE-301 will serve as the single efficacy study supporting our NDA submission.

Recent Highlights

- **Completed Initial Public Offering.** In May 2019, Milestone completed an initial public offering of 6,325,000 of its common shares at a public offering price of \$15.00 per share, including shares sold pursuant to the exercise by the underwriters of their option to purchase additional shares. Gross proceeds to Milestone were approximately \$95 million before deducting underwriting discounts, commissions and offering expenses.
- **Presented Studies on the Economic Impacts of PSVT at the ISPOR 2019 Annual Meeting.** In May 2019, Milestone presented data on the economic impact of PSVT and current standard of care therapy at the International Society for Pharmacoeconomics and Outcomes Research (ISPOR) 2019 Annual Meeting. Data demonstrated that current standards of care are both burdensome and costly, underscoring the need for a therapy, such as etripamil, that could potentially serve as an effective option for patients that addresses these issues.
- **Announced Additions to Board of Directors.** In April 2019, the Company announced the appointment of Paul Edick as Chairman of its Board of Directors. Mr. Edick currently serves as Chairman and Chief Executive Officer of Xeris Pharmaceuticals, a publicly traded specialty pharmaceutical company leveraging its novel technology platforms to develop and commercialize ready-to-use injectable and infusible drug formulations. Milestone also announced the appointment of Michael Tomsicek to its Board of Directors. Mr. Tomsicek is currently the Chief Financial Officer of CRISPR Therapeutics, a biopharmaceutical company focused on developing gene-based medicines.

Second Quarter 2019 Financial Results

- As of June 30, 2019, Milestone had cash, cash equivalents, and short-term investments of \$145.8 million and 24.5 million shares outstanding.
- Research and development expense for the second quarter of 2019 was \$10.5 million compared with \$2.6 million for the prior year period. For the six months ended June 30, 2019, research and development expense was \$18.3 million compared with \$5.6 million for the prior year period. The increase in 2019



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amounts reflects spending on Milestone's full Phase 3 clinical program evaluating etripamil for the treatment of PSVT.

- Commercial expense for the second quarter of 2019 was \$2.2 million compared with \$0.4 million for the prior year period. For the six months ended June 30, 2019, commercial expense was \$4.4 million compared with \$1.1 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.
- General and administrative expenses for the second quarter of 2019 were \$1.6 million compared with \$0.8 million for the prior year period. For the six months ended June 30, 2019, general and administrative expense was \$2.6 million compared with \$1.2 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.
- For the second quarter of 2019, operating loss was \$13.7 million compared to \$3.6 million in 2018. For the six months ended June 30, 2019, Milestone's operating loss was \$24.1 million compared to \$7.8 million in the prior year period.

Mid-Year Business Update Conference Call and Webcast

Milestone's management team will host a mid-year business update conference call and live audio webcast at 8:00 a.m. ET Tuesday, August 13, 2019, to discuss today's regulatory updates. To access the live call by phone, dial 800-529-3311 (domestic) or 470-495-9164 (international); the conference ID is 9498516. A live audio webcast of the event may also be accessed through the "Investors" section of Milestone's website at www.milestonepharma.com. A replay of the webcast will be available for 30 days following the event.

About Etripamil in Paroxysmal Supraventricular Tachycardia (PSVT)

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, 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. Etripamil is a novel, potent and short-acting calcium channel blocker designed by Milestone and being developed as a rapid-onset nasal spray to be administered by the patient to terminate episodes of PSVT as they occur. Milestone is actively recruiting patients for a Phase 3 clinical trial of etripamil for the treatment of PSVT. Milestone is also planning to initiate a Phase 2 clinical trial in the second half of 2019 in atrial fibrillation, another rapid heart rate condition, and expects to subsequently initiate an additional Phase 2 clinical trial in angina in 2020 to establish proof-of-concept for the broader use of etripamil.

Forward-Looking Statements:



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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 development of product candidates, (ii) the design, progress, timing, scope and results of clinical trials, (iii) the anticipated timing of disclosure of results of clinical trials, (iv) the potential benefits and success of the commercialization of product candidates, (v) the likelihood data will support future development and (vi) the likelihood of obtaining regulatory approval of Milestone's product candidates. Important factors that could cause actual results to differ materially from those in the forward-looking statements 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 March 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 STATEMENT OF OPERATIONS

(Unaudited)

	'000		'000	
[In US dollars]	Three months ended June 30,		Six months ended June 30,	
	2019	2018	2019	2018
	\$	\$	\$	\$
Operating expenses				
Research and development, net of tax credits	10,527	2,551	18,292	5,642
General and administrative	1,641	750	2,620	1,189
Commercial	2,166	375	4,352	1,100
Loss from operations	(14,334)	(3,676)	(25,264)	(7,931)
Interest income, net of bank charges	672	89	1,172	180
Loss and comprehensive loss before income taxes	(13,662)	(3,587)	(24,092)	(7,751)
Income tax (recovery) expense	(4)	2	18	18
Net loss and comprehensive loss for the period	(13,658)	(3,589)	(24,110)	(7,769)
Weighted average number of shares outstanding,				
basic and diluted	13,190,638	282,322	6,931,611	275,450
Net loss per share, basic and diluted	(1.04)	(12.71)	(3.48)	(28.20)



CONDENSED CONSOLIDATED BALANCE SHEET

(Unaudited)

[In US dollars]	'000	
	June 30, 2019	December 31, 2018
	\$	\$
ASSETS		
Current assets		
Cash, cash equivalents and short-term investments	145,824	85,976
Prepaid expenses and other current assets	5,727	2,075
Total current assets	151,551	88,051
Operating lease right-of-use asset	243	—
Property and equipment	43	30
Total assets	151,837	88,081
LIABILITIES		
Current liabilities		
Accounts payable and accrued liabilities	6,288	4,477
Current portion of operating lease liabilities	175	—
Income taxes payable	—	56
Total current liabilities	6,463	4,533
Operating lease liabilities	61	—
Total liabilities	6,524	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 and outstanding at June 30, 2019 and 596,787 shares issued and outstanding at December 31, 2018		
	226,211	2,039
Additional paid in Capital	3,116	2,655
Cumulative translation adjustment	(1,634)	(1,634)
Accumulated deficit	(82,380)	(58,270)
Total shareholders' equity (deficit)	145,313	(55,210)
Total liabilities and shareholders' equity	151,837	88,081



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