Milestone Pharmaceuticals Reports Second Quarter 2022 Financial Results and Provides Clinical and Corporate Update

August 10, 2022

-- Phase 3 RAPID study reached the target of 180 confirmed PSVT events treated with double-blind study medication required to initiate primary efficacy analysis; topline data readout for RAPID study remains on track for mid-second half 2022

-- Phase 3 PSVT study initiated in China

MONTREAL and CHARLOTTE, N.C., Aug. 10, 2022 /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 second quarter ended June 30, 2022, and provided a clinical and corporate update.

"Progress continues across our Phase 3 PSVT clinical program. Notably with the recent crossing of the 180th event in the RAPID trial, we now focus on the final stages of data cleaning and analysis and look forward to reporting top line data in the middle of the second half of this year," said Joseph Oliveto, President and Chief Executive Officer of Milestone Pharmaceuticals.

Recent Updates

- **Achieved Required Number of PSVT Events Treated with Double Blind Study Medication to Initiate Preparation for the Primary Efficacy Analysis for RAPID Trial; Company Remains on Track to Report RAPID Topline Data in Mid-Second Half 2022.** In July 2022, the RAPID trial, in which patients are randomized 1:1 to receive either etripamil or placebo, reached the pre-specified target of 180 confirmed PSVT events, adjudicated by a blinded, independent expert committee, required for the primary efficacy analysis. Data from RAPID is now in the process of being cleaned, verified, and analyzed, and the Company continues to expect to unblind RAPID results and report topline data in the middle of the second half of 2022. The primary efficacy analysis for the RAPID trial and the post hoc analysis of the NODE-301 trial will be time to conversion of supraventricular tachycardia (SVT) over the first 30 minutes following initial study drug administration. The RAPID and NODE-301 trials could potentially serve to fulfill the efficacy requirement for a future New Drug Application submission to the United States Food and Drug Administration for etripamil in patients with PSVT.
- **Milestone's Partner, Ji Xing Pharmaceuticals Limited, Enrolled First Patient in Phase 3 Study of Etripamil in China.** In July 2022, Milestone's partner, Ji Xing Pharmaceuticals Limited (Ji Xing), announced that it enrolled its first patient in its Phase 3 study of etripamil for the treatment of PSVT in China. The study is designed to evaluate the efficacy and safety of self-administered etripamil nasal spray as a treatment for PSVT with the eventual goal of generating clinical data potentially supportive of a new drug application in China. In May 2021, Milestone and Ji Xing entered into an exclusive license agreement to develop and commercialize etripamil for PSVT in Greater China.
- **Data from Phase 3 NODE-302 Open-Label Extension Study of Etripamil for the Treatment of PSVT Presented at Heart Rhythm 2022.** In April 2022, data from NODE-302, Milestone's Phase 3 open-label extension of the NODE-301 study evaluating a single, 70 mg dose of self-administered, intranasal etripamil in patients with PSVT, was presented at the Heart Rhythm Society's Heart Rhythm 2022 conference. The data demonstrated the potential for patients to self-treat recurrent SVT episodes with etripamil. Of 188 positively-adjudicated episodes observed in the trial, the PSVT conversion rate at 30 minutes following etripamil administration was 60.2%, and the need for emergency department (ED) intervention to terminate a PSVT episode was low (13% of patients and 8.5% of positively adjudicated PSVT episodes). Etripamil was generally well-tolerated, with adverse events (AEs) consistent with those observed in previous trials and largely confined to local and brief nasal AEs at the administration site. A copy of the presentation is available on request from Milestone Pharmaceuticals.
- **Hosted Virtual KOL Event on Etripamil for the Treatment of PSVT.** In April 2022, Milestone hosted a virtual Key Opinion Leader (KOL) event focused on etripamil for the possible treatment of PSVT. Members of management were joined by Bruce Stambler, M.D., FHRS, Director of Cardiac Arrhythmia Research and Education, Piedmont Heart Institute, Atlanta, GA, and Sean Pokorney, M.D., MBA, Director of the Arrhythmia Core Laboratory, Duke Clinical Research Institute, Assistant Professor of Medicine, Duke University School of Medicine, Durham, NC. The event featured an overview of PSVT, including disease prevalence, the current treatment landscape, patient and healthcare system burdens and a discussion of the potential commercial opportunity. A recording of the event is currently available under the News & Events of Milestone's website at www.milestonepharma.com.
- **New Clinical Analysis Evaluating the Drug Characteristics and Safety of Etripamil Presented at the American College of Cardiology (ACC) 71st Annual Scientific Session and Expo.** In April 2022, new analyses on the safety, tolerability, pharmacokinetics, and pharmacodynamics of etripamil in healthy Japanese and non-Japanese adults was presented at the ACC 71st Annual Scientific Session and Expo. The data demonstrate a comparable safety and tolerability profile in both Japanese and non-Japanese male and female adults, indicating no ethnic differences, and treatment-related

AEs consistent with the safety and tolerability profile of etripamil seen to date. A copy of the presentation is available in the Publications section of the Milestone Pharmaceuticals website.

Second Quarter 2022 Financial Results

- As of June 30, 2022, Milestone had cash, cash equivalents, and short-term investments of $86.2 million and 29.9 million common shares and 12.3 million common shares issuable upon exercise of pre-funded warrants outstanding.
- Research and development (R&D) expense for the second quarter of 2022 was $10.7 million, compared with $9.4 million for the prior year period. The difference is primarily the result of an increase in clinical personnel related costs, clinical consulting fees and contract research organization (CRO) costs due to advancing the RAPID Phase 3 efficacy and safety trial in etripamil for the treatment of PSVT. For the six months ended June 30, 2022, R&D expense was $19.4 million compared with $18.0 million for the prior year period. The $1.4 million increase in R&D expense in the six months ended June 30, 2022 is the result of clinical personnel related costs, clinical consulting fees and CRO costs due to advancing the RAPID Phase 3 efficacy and safety trial in etripamil for the treatment of PSVT. These increases were offset by lower drug formulation and manufacturing costs. Additionally, regulatory costs increased primarily due to personnel-related costs.
- General and administrative (G&A) expense for the second quarter of 2022 was $3.9 million, compared with $3.0 million for the prior year period. The difference is primarily the result of an increase in personnel-related costs and consulting fees for general and administrative expenses. For the six months ended June 30, 2022, G&A expense was $7.6 million compared with $5.7 million for the prior year period. The $1.9 million increase in G&A expense in the six months ended June 30, 2022 is primarily the result of personnel-related costs and consulting fees for general and administrative expenses.
- Commercial expense for the second quarter of 2022 was $2.2 million, compared with $1.8 million for the prior year period. The difference is primarily the result of an increase in consulting and marketing analytics. For the six months ended June 30, 2022, commercial expense was $3.9 million compared with $3.2 million for the prior year period. The $0.7 million increase in commercial expense in the six months ended June 30, 2022 is the result of personnel-related costs.
- For the second quarter of 2022, operating loss was $16.8 million, compared to operating income of $0.7 million for the prior year period. For the six months ended June 30, 2022, Milestone's operating loss was $30.9 million, compared to $11.9 million in the prior year period. Included in the operating income for the second quarter of 2021 and operating loss for the six months ended June 30, 2021 was primarily the one-time upfront payment of $15.0 million, recognized as revenue, generated from Milestone's License Agreement with Ji Xing.

About Paroxysmal Supraventricular Tachycardia

Paroxysmal supraventricular tachycardia (PSVT) is a condition characterized by intermittent episodes of a rapid heartbeat that starts and stops suddenly that affects approximately two million Americans. Episodes of supraventricular tachycardia (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 intravenous medications, including adenosine, beta-blockers and calcium channel blockers, have long been used for the acute treatment of PSVT. However, these medications must be administered under medical supervision, usually in an emergency department or other acute care setting.

About Atrial Fibrillation with Rapid Ventricular Rate

Atrial fibrillation (AFib) is a common arrhythmia marked by an irregular and often rapid heartbeat. AFib is estimated to affect five million patients in the United States, a prevalence projected by the Centers for Disease Control to increase to twelve million patients within the next 10 years. Atrial fibrillation with rapid ventricular rate (AFib-RVR) is a condition that some patients with AFib experience and includes episodes of abnormally high heart rate, often with symptoms such as palpitations, shortness of breath, dizziness and weakness. Oral calcium channel blockers and/or beta blockers are commonly used to reduce the heart rate in this condition. When AFib-RVR occurs, symptoms often cause patients to seek acute care in the emergency department, where standard-of-care procedures include intravenous administration of calcium channel blockers or beta blockers under medical supervision. Milestone's market research indicates that 30-40% of patients with AFib experience one or more symptomatic episodes of RVR per year that require treatment, suggesting a target addressable market of approximately three to four million patients in 2030 for etripamil in patients with AFib.

About Etripamil

Etripamil, a new chemical identity being Milestone's lead investigational product, is a novel calcium channel blocker designed to be a rapid-response therapy for episodic cardiovascular conditions. As a nasal spray that is self-administered by the patient, etripamil has the potential to shift the current treatment experience for many patients from the emergency department to a medically-unsupervised setting. Milestone is conducting a comprehensive development program for etripamil, with Phase 3 trials ongoing in paroxysmal supraventricular tachycardia (PSVT) and a Phase 2 proof-of-concept trial underway in patients with atrial fibrillation with rapid ventricular rate (AFib-RVR).

About Milestone Pharmaceuticals

Milestone Pharmaceuticals Inc. (Nasdaq: MIST), is a biopharmaceutical company focused on the development and commercialization of innovative cardiovascular medicines. Milestone's lead product candidate etripamil is currently in a Phase 3 clinical-stage program for the treatment of paroxysmal supraventricular tachycardia (PSVT) and in a Phase 2 proof-of-concept trial for the treatment of patients with atrial fibrillation with rapid ventricular rate (AFib-RVR). Milestone Pharmaceuticals operates in Canada and the United States. For more information, visit www.milestonepharma.com and follow Milestone 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 "believe," "will," "expect," "continue," "estimate," "potential," "progress" 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 to serve as a promising therapy for PSVT patients, the design, progress, timing, scope and results of the RAPID and ReVeRA trials; Milestone's ability to execute on the remainder of the PSVT program; the timing of release of unblind RAPID results and topline data with respect to the Company's RAPID trial; Milestone's ongoing plans to study etripamil in atrial fibrillation patients, the sufficiency of Milestone's current cash resources to support its operations, and estimates about the addressable market and commercial potential for treatments of atrial fibrillation with rapid ventricular rate. 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; risks and uncertainty related to the complexity inherent in cleaning, verifying and analyzing trial data; 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 the ongoing COVID-19 pandemic, 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 annual report on Form 10-K for the year ended December 31, 2021, under the caption "Risk Factors," as such discussion may be updated from time to time by subsequent filings we may make with the U.S. Securities & Exchange Commission. 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
(Unaudited, in thousands of US dollars, except share and per share data)

	Three months ended June 30,		Six months ended June 30,	
	2022	**2021**	**2022**	**2021**
Revenue	$ —	$ 15,000	$ —	$ 15,000
Operating expenses				
Research and development, net of tax credits	10,657	9,427	19,425	18,022
General and administrative	3,918	3,018	7,561	5,651
Commercial	2,231	1,843	3,867	3,209
Earnings (loss) from operations	(16,806)	712	(30,853)	(11,882)
Interest income, net	158	58	198	138
Net earnings (loss)	$ (16,648)	$ 770	$ (30,655)	$ (11,744)
Weighted average number of shares and pre-funded warrants outstanding, basic	42,278,563	41,673,370	42,260,682	41,465,961
Net earnings (loss) per share, basic	$ (0.39)	$ 0.02	$ (0.73)	$ (0.28)
Weighted average number of shares and pre-funded warrants outstanding, diluted	42,278,563	44,530,121	42,260,682	41,465,961
Net earnings (loss) per share, diluted	$ (0.39)	$ 0.02	$ (0.73)	$ (0.28)

CONSOLIDATED BALANCE SHEETS
(Unaudited, in thousands of US dollars, except share data)

	June 30, 2022	**December 31, 2021**
Assets		
Current assets		
Cash and cash equivalents	$ 63,237	$ 114,141
Short-term investment	23,000	—
Research and development tax credits receivable	539	356
Prepaid expenses	3,465	4,299
Other receivables	261	127

Total current assets	90,502	118,923
Operating lease assets	570	711
Property and equipment	227	215
Total assets	$ 91,299	$ 119,849
Liabilities, and Shareholders' Equity		
Current liabilities		
Accounts payable and accrued liabilities	$ 4,087	$ 6,551
Operating lease liabilities	169	224
Total current liabilities	4,256	6,775
Operating lease liabilities (net of current portion)	384	474
Total liabilities	4,640	7,249
Shareholders' Equity		
Common shares, no par value, unlimited shares authorized 30,005,884 shares issued and outstanding as of June 30, 2022, 29,897,559 shares issued and outstanding as of December 31, 2021	252,236	251,901
Pre-funded warrants - 12,327,780 issued and outstanding as of June 30, 2022 and 12,327,780 as of December 31, 2021	52,941	52,941
Additional paid-in capital	20,090	15,711
Cumulative translation adjustment	(1,634)	(1,634)
Accumulated deficit	(236,974)	(206,319)
Total shareholders' equity	86,659	112,600
Total liabilities and shareholders' equity	$ 91,299	$ 119,849

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