Milestone Pharmaceuticals Reports First Quarter 2024 Financial Results and Provides Regulatory and Corporate Update

May 13, 2024

– NDA for etripamil in PSVT resubmitted in 1Q 2024
– Cash resources as of March 31, 2024 expected to fund operations into 2026
– Dialogue with FDA to finalize Phase 3 protocol for etripamil in AFib-RVR is progressing

MONTREAL and CHARLOTTE, N.C., May 13, 2024 (GLOBE NEWSWIRE) -- Milestone Pharmaceuticals Inc. (Nasdaq: MIST) today reported financial results for the first quarter ended March 31, 2024 and provided a regulatory and corporate update.

"We're currently on track for the potential FDA approval of CARDAMYST™ (etripamil) nasal spray in the first half of 2025 to deliver a valuable treatment option to patients suffering from PSVT," said Joseph Oliveto, President and Chief Executive Officer of Milestone Pharmaceuticals. "We remain focused on working with FDA through the review process and preparing for commercialization and launch readiness. We expect that our current cash position, together with a potential future synthetic royalty payment, provides the needed resources to launch CARDAMYST in the U.S. market for patients suffering from PSVT."

First Quarter and Recent Program Updates

Etripamil for Patients with paroxysmal supraventricular tachycardia (PSVT)

- **Resubmitted a New Drug Application (NDA) for etripamil for PSVT.** The resubmission followed a Type A meeting held with the U.S. Food and Drug Administration (FDA) in February 2024, during which the Company worked to reach alignment with the Agency on resolution of the items raised in the Refusal to File letter. The resubmission package included restructured data sets that captured timing of reported adverse events in the Phase 3 PSVT trial and certain data files reformatted to facilitate the FDA's analyses. The Company currently expects a standard NDA review period.
- **New clinical data, demonstrating real-world use of etripamil for conversion of PSVT, were presented at The American College of Cardiology Scientific Sessions and published in the *Journal of the American College of Cardiology*.** The NODE-303 Phase 3 study (ClinicalTrials.gov ID NCT04072835) evaluated self-administered etripamil in an outpatient setting for up to multiple episodes of PSVT. The results indicated that symptom-prompted treatment with etripamil converted PSVT (restoring sinus rhythm) was 60.0% by 30 minutes after drug self-administration, and 69.9% by 60 minutes after drug self-administration; these rates of conversion were similar to those demonstrated in double-blinded and other open-label studies. Safety data were also consistent with prior studies' findings.

Etripamil for Patients with atrial fibrillation with a rapid ventricular rate (AFib-RVR)

- **Productive Phase 3 guidance received from FDA in 1Q 2024 meeting.** FDA reiterated its prior guidance on a single-study, supplemental New Drug Application pathway. The FDA further concurred with key study elements for Phase 3 including powering, inclusion criteria, and patient population, and offered clarification on the endpoints to guide study design. The Company will communicate further with the FDA in the second half of 2024, with the goal of finalizing the registrational study protocol.

Corporate Updates

- **In March 2024, closed a public offering of common shares and pre-funded warrants, raising net proceeds of approximately $32.2 million.** Milestone intends to use the proceeds from the Offering to continue the development and commercialization of etripamil in its lead indication of PSVT and its subsequent indication of AFib-RVR, as well as for working capital and other general corporate purposes.

First Quarter 2024 Financial Results

- As of March 31, 2024, Milestone had cash, cash equivalents, and short-term investments of $89.5 million, compared to $66.0 million as of December 31, 2023.
- There was no revenue for the first quarter ended March 31, 2024 compared to $1.0 million in the first quarter of 2023.
- Research and development expense for the first quarter of 2024 was $3.6 million, compared with $10.3 million for the prior year period. The decrease year over year was primarily due to the completion of the phase 3 clinical studies for PSVT in 2023.
- General and administrative expense for the first quarter of 2024 was $4.0 million, compared with $3.9 million for the prior year period.
- Commercial expense for the first quarter of 2024 was $2.9 million, compared with $2.4 million for the prior year period

- In connection with the revised timeline for the NDA submission, we undertook certain cash conservation measures. These cash conservation measures were substantially completed during the first quarter of 2024.
- For the first quarter of 2024, net loss was $10.4 million, compared to $15.0 million for the prior year period.

For further details on the Company's financials, refer to Form 10-Q for the quarter ended March 31, 2024, filed with the SEC on May 13, 2024.

About Paroxysmal Supraventricular Tachycardia

An estimated two million people in the United States are currently diagnosed with PSVT which is a type of arrhythmia or abnormal heart rhythm. PSVT is characterized by episodes of sudden onset rapid heartbeats often exceeding 150 to 200 beats per minute. The heart rate spike is unpredictable and may last several hours. The rapid heart rate often causes disabling severe palpitations, shortness of breath, chest discomfort, dizziness or lightheadedness, and distress, forcing patients to limit their daily activities. The uncertainty of when an episode of PSVT will strike or how long it will persist can provoke anxiety in patients and negatively impact their day-to-day life between episodes. The impact and morbidity from an attack can be especially detrimental in patients with underlying cardiovascular or medical conditions, such as heart failure, obstructive coronary disease, or dehydration. Many health care providers are dissatisfied with the lack of effective treatment options with patients often requiring prolonged, burdensome, and costly trips to the emergency department or even invasive cardiac ablation procedures.

About Atrial Fibrillation with Rapid Ventricular Rate

An estimated five million Americans suffer from atrial fibrillation (AFib), a common arrhythmia marked by an irregular, disruptive and often rapid heartbeat. The incidence of AFib is expected to grow to approximately 10 million by 2025 and up to about 12 million by 2030. A subset of patients with AFib experience episodes of abnormally high heart rate most often accompanied by palpitations, shortness of breath, dizziness, and weakness. While these episodes, known as AFib-RVR, may be treated by oral calcium channel blockers and/or beta blockers, patients frequently seek acute care in the emergency department to address symptoms. In 2016, nearly 800,000 patients were admitted to the emergency department due to AFib symptoms where treatment includes medically supervised intravenous administration of calcium channel blockers or beta blockers, or electrical cardioversion. With little available data for AFib-RVR, Milestone's initial market research indicates that 30 to 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-RVR.

About Etripamil

Etripamil is Milestone's lead investigational product. It is a novel calcium channel blocker nasal spray under clinical development for frequent and often highly symptomatic episodes of PSVT and AFib-RVR. It is designed as a self-administered rapid response therapy for patients thereby bypassing the need for immediate medical oversight. If approved, etripamil is intended to provide health care providers with a new treatment option to enable on-demand care and patient self-management. This portable, self-administered treatment may provide patients with active management and a greater sense of control over their condition. CARDAMYST™ (etripamil) nasal spray, the conditionally approved brand name for etripamil nasal spray, is well studied with a robust clinical trial program that includes a completed Phase 3 clinical-stage program for the treatment of PSVT and Phase 2 trial for the treatment of patients with AFib-RVR.

About Milestone Pharmaceuticals

Milestone Pharmaceuticals Inc. (Nasdaq: MIST) is a biopharmaceutical company developing and commercializing innovative cardiovascular solutions to improve the lives of people living with complex and life-altering heart conditions. The Company's focus on understanding unmet patient needs and improving the patient experience has led us to develop new treatment approaches that provide patients with an active role in self-managing their care. Milestone's lead investigational product is etripamil, a novel calcium channel blocker nasal spray that is being studied for patients to self-administer without medical supervision to treat symptomatic episodic attacks associated with PSVT and AFib-RVR.

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," "continue," "could," "demonstrate," "designed," "develop," "estimate," "expect," "may," "pending," "plan," "potential," "progress," "will", "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: our expected cash runway into 2026; the timing and outcomes of future interactions with U.S. and foreign regulatory bodies, including the FDA, including the timing of the FDA's review of the NDA; the timing of the receipt of the future synthetic royalty payment, if at all; our use of proceeds from the March 2024 private placement financing; the potential of etripamil to help patients living with these serious heart arrythmias; the continued ability of etripamil to achieve statistically superior ventricular rate reduction and improved symptom-relief when compared to placebo; the timing of the launch of etripamil; and the timing and outcomes of our clinical trials. Important factors that could cause actual results to differ materially from those in the forward-looking statements include, but are not limited to, whether our future interactions with the FDA will have satisfactory outcomes; whether and when, if at all, our NDA for etripamil will be approved by the FDA; whether the FDA will require additional trials or data which may significantly delay and put at risk our efforts to obtain approval and may not be successful, 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, evaluation and results of our 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, general economic, political, and market conditions, including deteriorating market conditions due to investor concerns regarding inflation, Russian hostilities in Ukraine and ongoing disputes in Israel and Gaza and overall fluctuations in the financial markets in the United States and abroad, risks related to pandemics and public health emergencies, and risks related the sufficiency of Milestone's capital resources and its ability to raise additional capital in the current economic climate. These and other risks are set forth in Milestone's filings with the U.S. Securities and Exchange Commission (SEC), including in its annual report on Form 10-K for the year ended March 31, 2023, under the caption "Risk Factors," as such discussion may be updated from time to time by subsequent filings Milestone may make with the SEC. 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.

Milestone Pharmaceuticals Inc.
Condensed Consolidated Statements of Loss (Unaudited)
(in thousands of US dollars, except share and per share data)

	Three months ended March 31,	
	2024	**2023**
Revenue	$ —	$ 1,000
Operating expenses		
Research and development, net of tax credits	3,639	10,257
General and administrative	3,953	3,889
Commercial	2,884	2,356
Loss from operations	(10,476)	(15,502)
Interest income	994	585
Interest expense	(872)	(33)
Net loss and comprehensive loss	$ (10,354)	$ (14,950)
Weighted average number of shares and pre-funded warrants outstanding, basic and diluted	50,155,111	42,853,275
Net loss per share, basic and diluted	$ (0.21)	$ (0.35)

Milestone Pharmaceuticals Inc.
Condensed Consolidated Balance Sheets (Unaudited)
(in thousands of US dollars, except share data)

	March 31, 2024	**December 31, 2023**
Assets		
Current assets		
Cash and cash equivalents	$ 10,131	$ 13,760
Short-term investments	79,350	52,243
Research and development tax credits receivable	711	643
Prepaid expenses	2,362	3,178
Other receivables	1,413	3,208
Total current assets	93,967	73,032
Operating lease right-of-use assets	1,785	1,917
Property and equipment	249	277
Total assets	$ 96,001	$ 75,226
Liabilities, and Shareholders' Equity		
Current liabilities		
Accounts payable and accrued liabilities	$ 3,575	$ 6,680
Operating lease liabilities	555	546
Total current liabilities	4,130	7,226
Operating lease liabilities, net of current portion	1,306	1,457
Senior secured convertible notes	50,644	49,772
Total liabilities	56,080	58,455
Shareholders' Equity		
Common shares, no par value, unlimited shares authorized 53,245,165 shares issued and outstanding as of March 31, 2024, 33,483,111 shares issued and outstanding as of December 31, 2023	287,879	260,504

Pre-funded warrants - 12,910,590 issued and outstanding as of March 31, 2024 and 9,577,257 as of December 31, 2023	53,076	48,459
Additional paid-in capital	35,346	33,834
Accumulated deficit	(336,380)	(326,026)
Total shareholders' equity	39,921	16,771
Total liabilities and shareholders' equity	$ 96,001	$ 75,226

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