# Milestone Pharmaceuticals Reports Fourth Quarter and Full Year 2020 Financial Results and Provides Clinical and Corporate Update

March 29, 2021

MONTREAL and CHARLOTTE, N.C., March 29, 2021 /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, 2020 and provided a clinical and corporate update.

"We are keenly focused on executing on our etripamil development program for patients with PSVT following clear regulatory guidance in the second half of 2020 and commencement of the pivotal Phase 3 RAPID study," said Joseph Oliveto, President and Chief Executive Officer of Milestone Pharmaceuticals. "We believe that etripamil has the potential to overcome the limitations of the current standard of care for PSVT, which is invasive, anxiety-provoking and costly, and to serve as a meaningful new therapeutic option for patients."

Mr. Oliveto added, "Beyond PSVT, we are pleased to announce that the Phase 2 ReVeRA study of etripamil in patients with atrial fibrillation and rapid ventricular rate is now underway. We are committed to assessing the full potential of etripamil and helping patients with episodic cardiovascular conditions, and we look forward to providing updates on our progress in the coming months."

**Recent Updates**

- **Company Continues to Guide to Topline Data from Pivotal Phase 3 RAPID Trial in Late 2021/Early 2022.** The pivotal Phase 3 RAPID trial, which is targeting a total of 180 adjudicated paroxysmal supraventricular tachycardia (PSVT) events, is expected to randomize approximately 500 patients 1:1 to receive either etripamil nasal spray or placebo. As previously announced, patients will be directed to administer a second dose of study drug if they do not experience symptom relief within 10 minutes of the first study drug administration to help maximize the potential treatment effect of etripamil. The primary efficacy analysis for both the RAPID trial and the already-completed NODE-301 trial will be time to conversion of SVT within 30 minutes following initial study drug administration, with a target p-value of less than 0.05 for each trial. The RAPID and NODE-301 trials could potentially serve to fulfill the efficacy requirement for a future New Drug Application (NDA) for etripamil in patients with PSVT.

  While clinical site initiations and patient enrollment have been impacted by COVID-19 pandemic-related factors, the Company has initiated mitigation activities including increasing the number of clinical sites and continues to guide toward topline data in late 2021/early 2022.

- **First Patient Enrolled in ReVeRA Phase 2 Proof-of-Concept Trial Evaluating Etripamil in Patients with Atrial Fibrillation and Rapid Ventricular Rate (AFib-RVR).** Milestone today announced that the first patient has been enrolled in ReVeRA, its Phase 2 proof-of-concept study of etripamil nasal spray in patients experiencing AFib-RVR. The Phase 2 double blind, placebo controlled, proof-of-concept study is designed to assess the safety and efficacy of etripamil nasal spray to reduce ventricular rate in patients with AFib-RVR experiencing an episode of elevated heart rate requiring treatment. The trial, which will be conducted in Canada in collaboration with the Montreal Heart Institute and other research centers, is expected to enroll approximately 50 patients randomized 1:1 to receive either 70 mg of etripamil nasal spray or placebo. The primary endpoint will assess reduction in ventricular rate, with key secondary endpoints including the time to achieve the maximum reduction in rate and duration of the effect.

- **Julia Gaebler, Ph.D., appointed as Vice President, Commercial Strategy and Portfolio Planning.** Today, Milestone announces the appointment of Julia Gaebler, Ph.D., as Vice President, Commercial Strategy and Portfolio Planning. Dr. Gaebler is a highly accomplished industry veteran with over 25 years of commercial strategy, market access, health services research, and corporate development experience. Dr. Gaebler joins Milestone from Health Advances, where she was Partner, and Head of Global Market Access, Pricing and Policy. Prior experience includes positions of increasing responsibility at Biogen, Amylin Pharmaceuticals, global and affiliate positions within Hoffman La Roche, and the RAND Corporation. She received her Ph.D. in Health Policy and Decision Sciences from Harvard University, M.A. in International Economics from Johns Hopkins University and B.A. in European Intellectual History from the University of Pennsylvania.

**Fourth Quarter and Full Year 2020 Financial Results**

- As of December 31, 2020, Milestone had cash, cash equivalents, and short-term investments of $142.3 million compared to $119.8 million as of December 31, 2019, and 29.8 million common shares and 11.4 million pre-funded warrants outstanding.

- Research and development expense for the fourth quarter of 2020 was $5.8 million compared with $14.1 million for the prior year period. For the full year ended December 31, 2020, research and development expense was $34.5 million compared with $42.0 million for the prior year. The COVID-19 pandemic contributed to delays in new clinical site initiation and patient enrollment, which translated into lower than expected research and development spending in the quarter and in the year ended December 31, 2020.
- General and administrative expense for the fourth quarter of 2020 was $1.7 million compared with $2.3 million for the prior year period. For the full year ended December 31, 2020, general and administrative expense was $10.3 million compared with $7.0 million for the prior year. The increase of general and administrative expense in the year is mainly attributable to higher insurance costs, additional headcount and non-cash compensation cost related to share-based compensation expense.
- Commercial expense for the fourth quarter of 2020 was $1.3 million compared with $2.5 million for the prior year period. For the full year ended December 31, 2020, commercial expense was $5.9 million compared with $8.9 million for the prior year. The decrease of commercial expense in the year ended December 31, 2020 reflects efforts in reducing operating expenses affecting primarily pre-commercialization activities as Milestone focused its efforts on an optimized clinical development pathway for etripamil.
- For the fourth quarter of 2020, operating loss was $8.8 million compared to $18.9 million for the prior year period. For the full year ended December 31, 2020, Milestone's operating loss was $50.0 million compared to $55.3 million for the prior year.
- The Company believes its current cash, cash equivalents, and short-term investments will be sufficient to fund anticipated operating expenses and capital expenditure requirements through 2022. This guidance includes anticipated costs associated with the Phase 3 RAPID trial, which are expected to increase as study enrollment progresses.

**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 which affects approximately two million Americans. 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, calcium channel blockers approved for the termination of SVT episodes must be administered intravenously under medical supervision, usually in an emergency department or other acute care setting.

**About Atrial Fibrillation and 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 and rapid ventricular rate (AFib-RVR) is a condition in which patients with AFib experience 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 manage heart rate in this condition. When episodes do occur, the corresponding symptoms often cause patients to seek care in the acute care setting such as the emergency department, where standard of care procedures include intravenous administration of calcium channel blockers or beta blockers under medical supervision. Milestone's initial qualitative market research indicates approximately 40% of patients with AFib experience one or more symptomatic episodes of AFib-RVR per year that require treatment, suggesting a target addressable market for etripamil in patients with AFib of approximately two million patients.

**About Etripamil**

Etripamil, 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 the at-home setting. Milestone is conducting a comprehensive development program for etripamil, with Phase 3 trials ongoing in paroxysmal supraventricular tachycardia (PSVT) and now a Phase 2 proof-of-concept trial underway in patients with atrial fibrillation and 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 and rapid ventricular rate (AFib-RVR). 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," "potential," "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 the potential of etripamil 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, Milestone's plans to study etripamil in atrial fibrillation patients, 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, 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 COVID-19, 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 quarterly report on Form 10-K for the year ended December 31, 2020, 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 COMPRENHENSIVE LOSS**
(Unaudited, in thousands of US dollars, except share and per share data)

| | Three months Ended December 31, | | Years Ended December 31, | |
|---|---|---|---|---|
| | **2020** | 2019 | **2020** | 2019 |
| | $ | $ | $ | $ |
| **Operating expenses** | | | | |
| Research and development, net of tax credits | **5,766** | 14,149 | **34,488** | 41,985 |
| General and administrative | **1,674** | 2,279 | **10,285** | 7,004 |
| Commercial | **1,322** | 2,464 | **5,937** | 8,892 |
| **Loss from operations** | **(8,762)** | (18,892) | **(50,710)** | (57,881) |
| Interest income, net of bank charges | **96** | 604 | **726** | 2,596 |
| **Loss before income taxes** | **(8,666)** | (18,288) | **(49,984)** | (55,285) |
| **Income tax recovery** | **—** | — | **(17)** | (56) |
| **Net loss and comprehensive loss for the period** | **(8,666)** | (18,288) | **(49,967)** | (55,229) |
| **Weighted average number of shares and pre-funded warrants outstanding, basic and diluted** | **38,424,384** | 24,496,347 | **29,344,993** | 15,784,750 |
| **Net loss per share, basic and diluted** | **(0.23)** | (0.75) | **(1.70)** | (3.50) |

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

| | **December 31, 2020** | December 31, 2019 |
|---|---|---|
| | **$** | $ |
| **ASSETS** | | |
| **Current Assets** | | |
| Cash, cash equivalents and short-term investments | **142,310** | 119,818 |
| Prepaid expenses and other current assets | **6,376** | 2,681 |
| **Total current assets** | **148,686** | 122,499 |
| Operating lease right-of-use asset | **980** | 524 |
| Property and equipment | **308** | 405 |
| **Total assets** | **149,974** | 123,428 |
| **LIABILITIES** | | |
| **Current liabilities** | | |
| Accounts payable and accrued liabilities | **5,914** | 7,997 |
| Current portion of operating lease liabilities | **245** | 330 |
| **Total current liabilities** | **6,159** | 8,327 |
| Operating lease liabilities | **696** | 184 |
| **Total liabilities** | **6,855** | 8,511 |
| **Shareholders' Equity** | | |
| Share capital | | |
| Common shares, no par value, unlimited shares authorized, 29,827,997 shares issued and outstanding as of December 31, 2020 and 24,505,748 shares issued and outstanding as of December 31, 2019 | **251,682** | 226,245 |
| Pre-funded Warrants - 11,417,034 issued and outstanding as of December 31, 2020 and nil at December 31, 2019 | **48,007** | - |
| Additional paid in capital | **8,530** | 3,805 |
| Cumulative translation adjustment | **(1,634)** | (1,634) |
| Accumulated deficit | **(163,466)** | (113,499) |
| **Total shareholders' equity** | **143,119** | 114,917 |
| **Total liabilities and shareholders' equity** | **149,974** | 123,428 |

**Contact:**

David Pitts
Argot Partners

212-600-1902
david@argotpartners.com

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