
**UNITED STATES
SECURITIES AND EXCHANGE COMMISSION**
Washington, D.C. 20549

FORM 8-K

CURRENT REPORT
Pursuant to Section 13 or 15(d) of the
Securities Exchange Act of 1934

Date of Report (Date of earliest event reported):
March 29, 2021

MILESTONE PHARMACEUTICALS INC.
(Exact name of registrant as specified in its charter)

Québec
(state or other jurisdiction of incorporation)

001-38899
(Commission File Number)

Not applicable
(I.R.S. Employer Identification No.)

**1111 Dr. Frederik-Philips
Boulevard, Suite 420
Montréal, Québec CA**
(Address of principal executive offices)

H4M 2X6
(Zip Code)

Registrant's telephone number, including area code: **(514) 336-0444**

(Former name or former address, if changed since last report.)

Check the appropriate box below if the Form 8-K filing is intended to simultaneously satisfy the filing obligation of the registrant under any of the following provisions (see General Instruction A.2. below):

- Written communications pursuant to Rule 425 under the Securities Act (17 CFR 230.425)
- Soliciting material pursuant to Rule 14a-12 under the Exchange Act (17 CFR 240.14a-12)
- Pre-commencement communications pursuant to Rule 14d-2(b) under the Exchange Act (17 CFR 240.14d-2(b))
- Pre-commencement communications pursuant to Rule 13e-4(c) under the Exchange Act (17 CFR 240.13e-4(c))

Securities registered pursuant to Section 12(b) of the Act:

Title of each class
Common Shares

Trading Symbol(s)
MIST

Name of each exchange on which registered
The Nasdaq Stock Market LLC

Indicate by check mark whether the registrant is an emerging growth company as defined in as defined in Rule 405 of the Securities Act of 1933 (§ 230.405 of this chapter) or Rule 12b-2 of the Securities Exchange Act of 1934 (§ 240.12b-2 of this chapter).

Emerging growth company

If an emerging growth company, indicate by check mark if the registrant has elected not to use the extended transition period for complying with any new or revised financial accounting standards provided pursuant to Section 13(a) of the Exchange Act.

Item 2.02. Results of Operations and Financial Condition.

On March 29, 2021, Milestone Pharmaceuticals Inc. (the “Company”) filed with the Securities and Exchange Commission (the “Commission”) its Annual Report on Form 10-K (the “Form 10-K”), which includes, at Item 8 thereof, its audited consolidated financial statements for the year ended December 31, 2020, and the report thereon of its Independent Registered Public Accounting Firm, PricewaterhouseCoopers LLP. (“PwC”), dated March 29, 2021.

Exhibit 99.1 to this Current Report on Form 8-K also includes the Company’s audited consolidated financial statements for the year ended December 31, 2020, and the report of PwC thereon. The sole difference between Exhibit 99.1 to this Current Report on Form 8-K and Item 8 to the Form 10-K is the inclusion in the audit report set forth in Exhibit 99.1 hereto of the Quebec professional permit number of the lead audit partner of PwC, which was intentionally omitted from the Report of Independent Registered Public Accounting Firm as included in the Form 10-K. The Company is furnishing this Current Report on Form 8-K in order to comply with the Quebec professional license requirements regulating chartered professional accountants of the Province of Quebec, Canada, as applicable to PwC.

The information contained in Item 2.02 and Exhibit 99.1 of this Current Report on Form 8-K is being furnished and shall not be deemed “filed” for purposes of Section 18 of the Securities Exchange Act of 1934, as amended, nor shall such information be deemed incorporated by reference in any filing under the Securities Act of 1933, as amended, except as shall be expressly set forth by specific reference in such filing.

Item 8.01. Other Events.

On March 29, 2021, the Company issued a press release announcing its financial results for the fourth quarter and fiscal year ended December 31, 2020 and providing a clinical and corporate update. A copy of the press release is attached as Exhibit 99.2 hereto and is incorporated herein by reference.

Item 9.01. Financial Statements and Exhibits.

(d) Exhibits.

Exhibit No.	Description
<u>99.1</u>	<u>Milestone Pharmaceuticals Inc.’s consolidated audited annual financial statements for the financial year ended December 31, 2020 and the Report of Independent Registered Public Accounting Firm thereon.</u>
<u>99.2</u>	<u>Press Release dated March 29, 2021.</u>

SIGNATURES

Pursuant to the requirements of the Securities Exchange Act of 1934, the registrant has duly caused this report to be signed on its behalf by the undersigned hereunto duly authorized.

MILESTONE PHARMACEUTICALS INC.

By: /s/ Amit Hasija
Amit Hasija
Chief Financial Officer

Dated: March 29, 2021

ITEM 8. FINANCIAL STATEMENTS**Report of Independent Registered Public Accounting Firm**

To the Shareholders and Board of Directors of Milestone Pharmaceuticals Inc.

Opinion on the Financial Statements

We have audited the accompanying consolidated balance sheets of Milestone Pharmaceuticals Inc. and its subsidiary (together, the Company) as of December 31, 2020 and 2019, and the related consolidated statements of loss and comprehensive loss, shareholders' equity and convertible preferred shares and of cash flows for the years then ended, including the related notes (collectively referred to as the consolidated financial statements). In our opinion, the consolidated financial statements present fairly, in all material respects, the financial position of the Company as of December 31, 2020 and 2019, and the results of its operations and its cash flows for the years then ended in conformity with accounting principles generally accepted in the United States of America.

Change in Accounting Principle

As discussed in Note 4 to the consolidated financial statements, the Company changed the manner in which it accounts for leases in 2019.

Basis for Opinion

These consolidated financial statements are the responsibility of the Company's management. Our responsibility is to express an opinion on the Company's consolidated financial statements based on our audits. We are a public accounting firm registered with the Public Company Accounting Oversight Board (United States) (PCAOB) and are required to be independent with respect to the Company in accordance with the U.S. federal securities laws and the applicable rules and regulations of the Securities and Exchange Commission and the PCAOB.

We conducted our audits of these consolidated financial statements in accordance with the standards of the PCAOB. Those standards require that we plan and perform the audit to obtain reasonable assurance about whether the consolidated financial statements are free of material misstatement, whether due to error or fraud. The Company is not required to have, nor were we engaged to perform, an audit of its internal control over financial reporting. As part of our audits we are required to obtain an understanding of internal control over financial reporting but not for the purpose of expressing an opinion on the effectiveness of the Company's internal control over financial reporting. Accordingly, we express no such opinion.

Our audits included performing procedures to assess the risks of material misstatement of the consolidated financial statements, whether due to error or fraud, and performing procedures that respond to those risks. Such procedures included examining, on a test basis, evidence regarding the amounts and disclosures in the consolidated financial statements. Our audits also included evaluating the accounting principles used and significant estimates made by management, as well as evaluating the overall presentation of the consolidated financial statements. We believe that our audits provide a reasonable basis for our opinion.

/s/ PricewaterhouseCoopers LLP¹
Montréal, Québec, Canada
March 29, 2021

We have served as the Company's auditor since 2016.

¹ CPA auditor, CA, public accountancy permit No.125677

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

	<u>December 31, 2020</u>	<u>December 31, 2019</u>
Assets		
Current assets		
Cash and cash equivalents	\$ 72,310	\$ 119,818
Short-term investments (note 3)	70,000	—
Research and development tax credits receivable	725	578
Prepaid expenses	5,428	1,845
Other receivables	223	258
Total current assets	<u>148,686</u>	<u>122,499</u>
Operating lease right-of-use assets (note 4)	980	524
Property and equipment (note 5)	308	405
Total assets	<u>\$ 149,974</u>	<u>\$ 123,428</u>
Liabilities		
Current liabilities		
Accounts payable and accrued liabilities (note 6)	\$ 5,914	\$ 7,997
Current portion of operating lease liabilities	245	330
Total current liabilities	<u>6,159</u>	<u>8,327</u>
Operating lease liabilities	696	184
Total liabilities	<u>6,855</u>	<u>8,511</u>
Shareholders' Equity (note 1, note 7)		
Share capital		
Common shares, no par value, unlimited shares authorized, 29,827,997 shares issued and outstanding as of December 31, 2020, 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 at December 31, 2020, 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	<u>143,119</u>	<u>114,917</u>
Total liabilities and shareholders' equity	<u>\$ 149,974</u>	<u>\$ 123,428</u>

The accompanying notes are an integral part of these consolidated financial statements.

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

	Year ended December 31,	
	2020	2019
Operating expenses		
Research and development, net of tax credits (note 10)	\$ 34,488	\$ 41,985
General and administrative	10,285	7,004
Commercial	5,937	8,892
Loss from operations	\$ (50,710)	\$ (57,881)
Interest income, net of bank charges	726	2,596
Loss before income taxes	(49,984)	(55,285)
Income tax recovery (note 9)	(17)	(56)
Net loss and comprehensive loss for the year	\$ (49,967)	\$ (55,229)
Weighted average number of shares and pre-funded warrants outstanding, basic and diluted	29,344,993	15,784,750
Net loss per share, basic and diluted (note 8)	\$ (1.70)	\$ (3.50)

The accompanying notes are an integral part of these consolidated financial statements.

Milestone Pharmaceuticals Inc.
Consolidated Statements of Shareholders' Equity and Convertible Preferred Shares
(in thousands of US dollars, except share data)

	Common Shares		Convertible Preferred Shares												Pre-funded warrants		Additional paid-in capital	Cumulative translation adjustment	Accumulated deficit	Total
			Class A1		Class A2		Class B		Class C		Class D1		Class D2							
	Number of shares	Amount	Number of shares	Amount	Number of shares	Amount	Number of shares	Amount	Number of shares	Amount	Number of shares	Amount	Number of shares	Amount	Number of warrants	Amount				
Balance as of December 31, 2018	596,787	\$ 2,039	372,211	\$ 2,027	2,443,914	\$ 12,643	2,830,907	\$ 17,198	3,786,878	\$ 27,236	6,893,236	64,719	1,223,656	14,935	—	\$ —	\$ 2,655	\$ (1,634)	\$ (58,270)	\$ 83,548
Transactions in 2019																				
Net loss and comprehensive loss	—	—	—	—	—	—	—	—	—	—	—	—	—	—	—	—	—	—	(55,229)	(55,229)
Exercise of stock options (note 7)	33,159	85	—	—	—	—	—	—	—	—	—	—	—	—	—	—	(41)	—	—	44
Share-based compensation (note 7)	—	—	—	—	—	—	—	—	—	—	—	—	—	—	—	—	1,191	—	—	1,191
Initial public offering (note 7)	6,325,000	85,363	—	—	—	—	—	—	—	—	—	—	—	—	—	—	—	—	—	85,363
Preferred share conversion (note 7)	17,550,802	138,758	(372,211)	(2,027)	(2,443,914)	(12,643)	(2,830,907)	(17,198)	(3,786,878)	(27,236)	(6,893,236)	(64,719)	(1,223,656)	(14,935)	—	—	—	—	—	—
Balance as of December 31, 2019	<u>24,505,748</u>	<u>\$226,245</u>	<u>—</u>	<u>\$ —</u>	<u>—</u>	<u>\$ —</u>	<u>—</u>	<u>\$ —</u>	<u>—</u>	<u>\$ —</u>	<u>—</u>	<u>\$ —</u>	<u>—</u>	<u>\$ —</u>	<u>—</u>	<u>\$ —</u>	<u>3,805</u>	<u>\$ (1,634)</u>	<u>\$ (113,499)</u>	<u>\$114,917</u>
Transactions in 2020																				
Net loss and comprehensive loss	—	—	—	—	—	—	—	—	—	—	—	—	—	—	—	—	—	—	(49,967)	(49,967)
Exercise of stock options (note 7)	226,352	520	—	—	—	—	—	—	—	—	—	—	—	—	—	—	(220)	—	—	300
Share-based compensation (note 7)	—	—	—	—	—	—	—	—	—	—	—	—	—	—	—	—	4,945	—	—	4,945
Private Placement (note 7)	—	—	—	—	—	—	—	—	—	—	—	—	—	—	—	—	6,655,131	24,771	—	24,771
Public Offering	5,095,897	24,917	—	—	—	—	—	—	—	—	—	—	—	—	—	—	4,761,903	23,236	—	48,153
Balance as of December 31, 2020	<u>29,827,997</u>	<u>\$251,682</u>	<u>—</u>	<u>\$ —</u>	<u>—</u>	<u>\$ —</u>	<u>—</u>	<u>\$ —</u>	<u>—</u>	<u>\$ —</u>	<u>—</u>	<u>\$ —</u>	<u>—</u>	<u>\$ —</u>	<u>11,417,034</u>	<u>\$48,007</u>	<u>\$ 8,530</u>	<u>\$ (1,634)</u>	<u>\$ (163,466)</u>	<u>\$143,119</u>

The accompanying notes are an integral part of these consolidated financial statements.

Milestone Pharmaceuticals Inc.
Consolidated Statements of Cash Flows
(in thousands of US dollars)

	Year ended December 31,	
	2020	2019
Cash flows from		
Operating activities		
Net loss for the year	\$ (49,967)	\$ (55,229)
Adjustments to reconcile net loss to net cash used in operating activities:		
Amortization of property and equipment (note 5)	97	38
Share-based compensation expense (note 7)	4,945	1,191
Changes in operating assets and liabilities:		
Other receivables	35	119
Research and development tax credits receivable	(147)	(288)
Prepaid expenses	(3,583)	(447)
Operating lease right of use asset, net	(29)	—
Accounts payable and accrued liabilities	(2,083)	3,520
Income taxes payable (receivable)	—	(56)
Net cash used in operating activities	<u>(50,732)</u>	<u>(51,152)</u>
Investing Activities		
Acquisition of property and equipment	—	(413)
Acquisition of short-term investments	(90,000)	(35,000)
Maturity of short-term investments	20,000	35,029
Net cash used in investing activities	<u>(70,000)</u>	<u>(384)</u>
Financing activities		
Net proceeds from issuance of common shares in Initial Public Offering	—	85,363
Issuance of common shares on exercise of share options (note 7)	300	44
Net proceeds from issuance of common shares in a public offering (note 7)	24,917	—
Net proceeds from issuance of pre-funded warrants in a public offering (note 7)	23,236	—
Net proceeds from issuance of pre-funded warrants in a private placement (note 7)	24,771	—
Net cash provided by financing activities	<u>73,224</u>	<u>85,407</u>
Net increase (decrease) in cash and cash equivalents during the year	(47,508)	33,871
Cash and cash equivalents – Beginning of year	119,818	85,947
Cash and cash equivalents – End of year	<u>\$ 72,310</u>	<u>\$ 119,818</u>

The accompanying notes are an integral part of these consolidated financial statements.

1 Organization and nature of operations

Milestone Pharmaceuticals Inc. (Milestone or the Company) is a biopharmaceutical company incorporated under the Business Corporations Act of Québec. Milestone is focused on the development and commercialization of innovative cardiovascular medicines. Milestone's lead product candidate, etripamil, is a novel, potent short-acting calcium channel blocker that the Company designed and is developing as a rapid-onset nasal spray to be administered by patients. The Company is developing etripamil to treat paroxysmal supraventricular tachycardia, atrial fibrillation, and other cardiovascular indications.

2 Summary of significant accounting policies

a) Basis of consolidation

The consolidated financial statements include the accounts of the Company and Milestone Pharmaceuticals USA, Inc. Milestone Pharmaceuticals USA, Inc. began its operations on March 3, 2017. All intercompany transactions and balances have been eliminated.

b) Basis of presentation and use of accounting estimates

These consolidated financial statements of the Company have been presented in United States dollars (USD) and have been prepared in accordance with accounting principles generally accepted in the United States of America (U.S. GAAP), including the applicable rules and regulations of the Securities and Exchange Commission (SEC) regarding financial reporting.

The preparation of consolidated financial statements in conformity with US GAAP requires the Company to make estimates and judgments that affect certain reported amounts of assets and liabilities and disclosure of contingent assets and liabilities at the date of the consolidated financial statements and the reported amounts of revenue and expenses during the period. The Company bases its estimates and assumptions on current facts, historical experience and various other factors that it believes are reasonable under the circumstances, to determine the carrying values of assets and liabilities that are not readily apparent from other sources. Significant estimates and judgments include, but are not limited to, research and development tax credits recoverable, progress of activities performed by the CROs and CMOs which are used to calculate the research and development expense incurred, and share-based compensation. Accordingly, actual results may differ from those estimates and such differences may be material.

The COVID-19 pandemic has had an impact on the Company's business, operations and clinical development timelines. Government orders and restrictions in order to control the spread of the disease have impacted patient recruitment, enrollment and follow-up visits at clinical sites. The Company will continue to evaluate the COVID-19 pandemic impact on the development timelines of its clinical programs. Estimates and assumptions about future events and their effects cannot be determined with certainty and therefore require the exercise of judgment. As of the date of issuance of these consolidated financial statements, the Company is not aware of any specific event or circumstance that would require the Company to update its estimates, assumptions and judgments. These estimates may change as new events occur and additional information is obtained and are recognized in the consolidated financial statements as soon as they become known. Actual results could differ from those estimates and any such differences may be material to the Company's consolidated financial statements.

c) Segment information

The Company manages its operations as a single operating segment for the purposes of assessing performance and making operating decisions while focusing on the development and commercialization of innovative cardiovascular medicines.

d) Cash and cash equivalents

Cash and cash equivalents consist of cash and highly liquid investments that are readily convertible into cash with original maturities of three months or less at acquisition date.

e) Short term investments

Short term investments are classified as held-to-maturity, are initially recognised at fair value and are subsequently accounted for at amortized cost. They are comprised of guaranteed investment certificates with a maturity greater than 90 days but less than one year and, as such, are classified as current assets.

f) Concentration of credit risk

Financial instruments which potentially subject the Company to concentration of credit risk consist primarily of cash and cash equivalents and investment securities classified as held to maturity. The Company maintains deposits in financial institutions. Management believes that the Company is not exposed to significant credit risk due to the financial position of the depository institutions in which those deposits are held. Additionally, the Company has adopted an investment policy that includes guidelines relative to credit quality, diversification of maturities and liquidity.

g) Currency Risk

The Company is exposed to currency risk due to financial instruments denominated in foreign currencies. The Company is exposed to the Canadian dollar currency risk and does not enter into arrangements to hedge its currency risk exposure.

h) Property and equipment

Property and equipment is stated at historical cost less accumulated amortization. Expenditures for maintenance and repairs are recorded to expense as incurred. The Company reviews its property and equipment whenever events or changes in circumstances indicate that the carrying value of certain assets might not be recoverable and recognizes an impairment loss when it is probable that an asset's realizable value is less than the carrying value. To date, no such impairment losses have been recorded. Amortization is calculated using the straight-line method over the following estimated useful lives of the assets:

Computer hardware and software	3 years
Office equipment	5 years
Furniture and fixtures	5 years
Leasehold improvements	over the lease-term

i) Leases

Effective January 1, 2019, the Company adopted ASC 842, Leases (ASC 842), using the required modified retrospective approach and utilizing the effective date as its date of initial application.

At the inception of an arrangement, the Company determines whether the arrangement is or contains a lease based on the unique facts and circumstances present in the arrangement. Leases with a term greater than one year are recognized on the balance sheet as right-of-use assets and short-term and long-term lease liabilities, as applicable. The Company does not have financing leases.

Operating lease liabilities and their corresponding right-of-use assets are initially recorded based on the present value of lease payments over the expected remaining lease term. Right-of-use assets are subsequently accounted for as long-lived assets, including evaluating for indicators of impairment. Certain adjustments to the right-of-use asset may be required for items such as incentives received. The interest rate implicit in lease contracts is typically not readily determinable. As a result, the Company utilizes its incremental borrowing rate to discount lease payments, which reflects the fixed rate at which the Company could borrow on a collateralized basis the amount of the lease payments in the same currency, for a similar term, in a similar economic environment. Prospectively, the Company will adjust the right-of-use assets for straight-line rent expense or any incentives received and remeasure the lease liability at the net present value using the same incremental borrowing rate that was in effect as of the lease commencement or transition date.

The Company has elected not to recognize leases with an original term of one year or less on the balance sheet. The Company typically only includes an initial lease term in its assessment of a lease arrangement. Options to renew a lease are not included in the Company's assessment unless there is reasonable certainty that the Company will renew.

j) Pre-funded warrants

Pre-funded warrants allow the holder to pay little or no consideration to receive the shares upon exercise of the warrant. The pre-funded warrants do not meet the definition of a derivative under ASC 815 because their fair value at issuance is equal to the fair value of the shares underlying the warrant. As such, they have the characteristics of a prepaid forward sale of equity. As a result, the pre-funded warrants are accounted for as equity instruments.

k) Share issuance costs

Share issuance costs applicable to the issuance of equity instruments are recorded as a reduction of the financing equity proceeds.

l) Research and development and investment tax credits

Research and development costs are charged to expense as costs are incurred in performing research and development activities. The Company's research and development costs consist primarily of salaries and fees paid to contract research organizations (CROs) and to contract manufacturing organizations (CMOs).

Clinical trial expenses include direct costs associated with CROs, direct CMO costs for the formulation and packaging of clinical trial material, as well as investigator and patient related costs at sites at which the Company's trials are being conducted. Direct costs associated with the Company's CROs and CMOs are generally payable on a time and materials basis, or when milestones are achieved. The invoicing from clinical trial sites can lag several months. The Company records expenses for its clinical trial activities performed by third parties based upon estimates of the percentage of work completed of the total work over the life of the individual study in accordance with agreements established with CROs and clinical trial sites. The Company determines the estimates through discussions with internal clinical personnel, CROs and CMOs as to the progress or stage of completion of trials or services and the agreed upon fee to be paid for such services based on facts and circumstances known to the Company as of each consolidated balance sheet date. The actual costs and timing of clinical trials are highly uncertain, subject of risks and may change depending upon a number of factors, including the Company's clinical development plan. If the actual timing of the performance of services of the level of effort varies from the estimate, the Company will adjust the accrual accordingly.

The Company recognizes the benefit of Canadian research and development tax credits as a reduction of research and development costs for fully refundable investment tax credits and as a reduction of income taxes for investment tax credits that can only be claimed against income taxes payable when there is reasonable assurance that the claim will be recovered.

m) Income taxes

The provision for income taxes is computed using the liability method. Under this method, deferred tax assets and liabilities are determined based on differences between financial reporting and tax bases of assets and liabilities. Deferred tax assets and liabilities are measured using enacted tax rates and laws that will be in effect when the differences are expected to reverse. A valuation allowance is recorded to reduce the carrying amount of deferred income tax assets when it is more likely than not that these assets will not be realized. Tax benefits related to tax positions not deemed to meet the "more-likely-than-not" threshold are not permitted to be recognized in the consolidated financial statements.

n) Foreign currency translation and transactions

The functional currency of the Company is the US dollar. Accordingly, transactions denominated in currencies other than the functional currency are measured and recorded in the functional currency at the exchange rate in effect on the date of the transactions. At each consolidated balance sheet date, monetary assets and liabilities denominated in currencies other than the functional currency are remeasured using the exchange rate in effect at that date. Non-monetary assets and liabilities and revenue and expense items denominated in foreign currencies are translated into the functional currency using the exchange rate prevailing at the dates of the respective transactions. Any gains or losses arising on remeasurement are included in the consolidated statement of operations.

o) Share based compensation

The Company has a share based compensation plan which is described in detail in note 7 and records all share-based payments, including grants of employee share options, at their fair values. The fair value of share options granted to employees and non-employees is estimated at the date of grant using the Black-Scholes option pricing model. The Company recognizes share based compensation expense over the requisite service period of the individual grants, which equals the vesting period, using the straight-line method. Forfeitures, if any, are recorded as they occur. Any consideration paid by employees on exercising share options and the corresponding portion previously credited to contributed surplus are credited to share capital. The Black-Scholes option pricing model used by the Company to calculate option values was developed to estimate fair value.

The Company approved an employee share purchase plan in April 2019, which became effective on May 8, 2019 and is described in note 7. The plan provides a means by which eligible employees of the company and certain designated companies may be given an opportunity to purchase common shares. The plan permits the company to grant a series of purchase rights to eligible employees under an employee stock purchase plan.

p) Recently adopted accounting pronouncements

New Accounting Policies - Financial Instruments - Credit Losses

In June 2016, the Financial Accounting Standards Board issued Accounting Standards Update 2016-13, Financial Instruments - Credit Losses (Topic 326): Measurement of Credit Losses on Financial Instruments (ASU 2016-13). ASU 2016-13 significantly changes the impairment model for most financial assets and certain other instruments. ASU 2016-13 will require immediate recognition of estimated credit losses expected to occur over the remaining life of many financial assets, which will generally result in earlier recognition of allowances for credit losses on loans and other financial instruments. The Company adopted ASU 2016-13 effective January 1, 2020 and the adoption did not have at the measurement of credit losses.

q) Significant Risks and Uncertainties

The COVID-19 pandemic has had an impact on our business, operations and clinical development timelines. Government orders and restrictions in order to control the spread of the disease have impacted patient recruitment, enrollment and follow-up visits at clinical sites. With the global spread of the ongoing COVID-19 pandemic, the Company has implemented business continuity plans designed to address and mitigate the impact of the COVID-19 pandemic on its business. The Company anticipates that the COVID-19 pandemic will continue to have an impact on the development timelines for its clinical programs. The extent to which the COVID-19 pandemic continues to impact its business, its clinical development and regulatory efforts, its corporate development objectives and the value of and market for its common shares will depend on future developments that remain highly uncertain and cannot be predicted with confidence at this time, such as the ultimate duration of the pandemic, travel restrictions, quarantines, social distancing and business closure requirements in the U.S., Europe and other countries, and the effectiveness of actions taken globally to contain and treat the disease. The global economic slowdown, the overall disruption of global healthcare systems and the other risks and uncertainties associated with the pandemic could have a material adverse effect on the Company's business, financial condition, results of operations and growth prospects.

In addition, the Company is subject to other challenges and risks specific to its business and its ability to execute on its strategy, as well as risks and uncertainties common to companies in the pharmaceutical industry, including, without limitation, risks and uncertainties associated with: obtaining regulatory approval of its product candidate; delays or problems in the supply of its study drug or failure to comply with manufacturing regulations; identifying, acquiring or in-licensing product candidates; pharmaceutical product development and the inherent uncertainty of clinical success; and the challenges of protecting and enhancing its intellectual property rights; and complying with applicable regulatory requirements.

r) Sources of Liquidity and Funding Requirements

Since inception, the Company incurred significant operating losses. Prior to May 2019, the Company financed its operations primarily through sales of convertible preferred shares to accredited investors generating net proceeds of \$138.8 million. In May 2019, the Company received net proceeds of \$85.4 million from its Initial Public Offering (IPO). In July 2020, the Company received \$24.8 million of net proceeds from the private placement of pre-funded warrants to existing shareholders (note 7). In October 2020, the Company concluded an offering of common shares and pre-funded warrants for net proceeds of \$48.2 million (note 7).

The Company has incurred operating losses and experienced negative operating cash flows since its inception and anticipates to continue to incur losses for at least the next several years. As of December 31, 2020, the Company had cash, cash equivalents and short-term investments of \$142.3 million and an accumulated deficit of \$163.5 million.

3 Short-term investments

Short-term investments are comprised of term deposits issued in US currency, earning interest between 0.30% and 0.86%, maturing between January 29, 2021 and August 16, 2021. These short-term investments are in scope of ASC 320, Investments - Debt Securities. The short-term investments maturity is greater than 90 days but less than one year, and they are classified as held to maturity, recorded as current assets and are accounted for at amortized cost.

4 Leases

On June 3, 2019, the Company entered into a new lease arrangement for a three-year term for its office located in Charlotte, NC. The Company recognized the operating lease right-of-use asset and operating lease liabilities at the lease commencement date on September 10, 2019. The interest rate implicit in lease contracts is not readily determinable and the Company does not have a public credit rating and carries no debt. As such, several factors were considered in the determination of the Company's incremental borrowing rate used in determining the present value of lease payments. The Company's examined credit ratings for similar companies, assumed equivalency between the Canadian and U.S. markets for collateralized debt and used rates over the 36-month period. This resulted in an incremental borrowing rate of 8%. Lease expenses are recognized on a straight-line basis over the lease term, which is accomplished by increasing the amortization of the right-of-use asset as interest expense on the lease liability declines over the lease term. The company was not reasonably certain of renewing the lease following the initial term and recognized the right-of-use asset and operating lease liabilities over the 36-month period ending September 30, 2022.

On July 1, 2020, the Company entered into an arrangement for the lease renewal for its headquarters located in Ville Saint-Laurent, Quebec. The 5-year lease term is from December 1, 2020 expiring on November 30, 2025. The Company revalued the operating lease right-of-use asset and operating lease liabilities at the effective lease arrangement date of July 1, 2020. The Company's examined credit ratings for similar companies, assumed equivalency between the Canadian and U.S. markets for collateralized debt and used rates for the remaining lease term of 65 months. This resulted in an incremental borrowing rate of 5.26%. Lease expenses are recognized on a straight-line basis over the lease term, which is accomplished by increasing the amortization of the right-of-use asset as interest expense on the lease liability declines over the lease term. The Company is not reasonably certain of renewing the lease following the current renewal option and recognized the right-of-use asset and operating lease liabilities to November 30, 2025.

The Company's two operating office leases right-of-use assets as at December 31 were as follows:

	2020	2019
Opening balance	\$ 524	\$ 321
New operating lease right-of-use asset	—	401
Right-of-use adjustment renewal on July 1, 2020	735	—
Amortization of right-of-use asset	(279)	(198)
Closing balance	<u>\$ 980</u>	<u>\$ 524</u>

Operating lease expenses of \$318 are included in general and administrative operating expenses in the consolidated statement loss and comprehensive loss, and within operating activities in the statement of cash flows for the year ended December 31, 2020 [2019 - \$277], and are comprised of two operating lease right-of-use assets and one operating lease of less than 12 months.

The following table summarizes the future minimum lease payments of right-of-use assets operating lease as at December 31, 2020:

January 1, 2021 to December 31, 2021	\$ 293
January 1, 2022 to December 31, 2022	255
January 1, 2023 to December 31, 2023	175
January 1, 2024 to December 31, 2024	175
January 1, 2025 to November 30, 2025	159
	<u>1,057</u>
Less interest	(115)
	<u>\$ 942</u>

5 Property and equipment

Property and equipment consist of the following at December 31:

	2020	2019
Computer hardware and software	\$ 22	\$ 22
Office equipment	406	406
Leasehold improvements	26	26
Total	\$ 454	\$ 454
Less accumulated depreciation and amortization	(146)	(49)
Property and equipment, net	<u>\$ 308</u>	<u>\$ 405</u>

During the year ended December 31, 2020 and December 31, 2019, the Company did not record any write off. For the year ended December 31, 2020, amortization expense was \$97 [2019-\$38] and was included in research and development expense.

6 Accounts payable and accrued liabilities

Accounts payable and accrued liabilities comprised the following as of December 31:

	2020	2019
Trade accounts payable	\$ 4,641	\$ 4,376
Accrued research and development liabilities	152	1,513
Other accrued liabilities	164	331
Accrued compensation and benefits payable	957	1,777
	<u>\$ 5,914</u>	<u>\$ 7,997</u>

7 Shareholders' equity

Authorized share capital

An unlimited number of common shares, voting and participating, without par value.

In May 2019, the Company completed its initial public offering (IPO). Upon the closing of the IPO, all outstanding redeemable convertible preferred shares of Class A1, A2, B, C, D1 and D2 (collectively known as Convertible Preferred Shares) converted into 17,550,802 common shares.

As of December 31, 2020, 523,821 common shares were available under the Employee Stock Purchase Plans (ESPP) and no common shares have been issued.

During the year ended December 31, 2020, the Company issued a total of 226,352 common shares [2019 - 33,162] for a total cash consideration of \$300 [2019 - \$44] pursuant to the exercise of stock options at an average exercise price of \$1.33 per share [2019 - \$1.33]. As a result, an amount of \$220 [2019 - \$41] previously included in additional paid-in capital related to the exercised options has been credited to share capital and deducted from additional paid-in capital.

Pre-funded warrants – Private Placement

On July 23, 2020, the Company entered into a securities purchase agreement to sell and issue in a private placement pre-funded warrants of 6,655,131 of the Company's common shares, at a purchase price of \$3.7465 per pre-funded warrant for aggregate net proceeds of \$24.8 million (the Private Placement). The Private Placement closed on July 24, 2020. Each pre-funded warrant is exercisable for one of the Company's common shares at an exercise price of \$0.01 per share, has no expiration date, and is immediately exercisable, subject to certain beneficial ownership limitations. The pre-funded warrants are classified and accounted for as equity.

Open Market Sale Agreement

On July 29, 2020, the Company entered into an Open Market Sale AgreementSM with respect to an at-the-market offering program (ATM Program) under which the Company may issue and sell its common shares having an aggregate offering price of up to \$50 million. The Company has not sold shares under the ATM program as of the date of this filing.

Pre-funded warrants and common shares – Public offering

On October 22, 2020, the Company issued (i) 5,095,897 common shares, without par value, at a price to the public of \$5.25 per share, and (ii) pre-funded warrants to purchase 4,761,903 common shares at an exercise price equal to \$0.01 per share, at a price to the public of \$5.24 per common share underlying the pre-funded warrants (the Offering). The net proceeds to the Company from the Offering were \$48.2 million. The pre-funded warrants are classified and accounted for as equity.

Additional paid-in capital

	2020	2019
Opening balance	\$ 3,805	\$ 2,655
Share-based compensation expense	4,945	1,191
Exercise of stock options	(220)	(41)
Closing balance	<u>\$ 8,530</u>	<u>\$ 3,805</u>

Share-based compensation

The Company's board of directors adopted and its shareholders approved the 2019 Equity Incentive Plan (the 2019 Plan) in April 2019, which became effective on May 8, 2019 in connection with the IPO. Initially, the maximum number of the Company's common shares that may be issued under the 2019 Plan was 4,710,564 shares, which is the sum of (1) 1,923,501 new shares, plus (2) the number of shares (not to exceed 2,787,063 shares) (i) that remained available for the issuance of awards under the Company's Stock Option Plan (the "2011 Plan") at the time the 2019 Plan became effective, and (ii) any shares subject to outstanding options or other share awards that were granted under the 2011 Plan that terminate, expire or are otherwise forfeited, reacquired or withheld. In addition, the number of the Company's common shares reserved for issuance under the 2019 Plan will automatically increase on January 1 of each calendar year, starting on January 1, 2020 through January 1, 2029, in an amount equal to 4% of the total number of the Company's capital shares outstanding on the last day of the calendar month before the date of each automatic increase, or a lesser number of shares determined by the Company's board of directors. As of May 8, 2019, the Company's 2011 Plan was terminated and no further option grants will be made under the 2011 Plan.

Under the 2019 Plan and 2011 Plan, unless otherwise decided by the Board of Directors, options vest and are exercisable as follows: 25% vest and are exercisable on the one year anniversary of the grant date and one thirty-sixth (1/36th) of the remaining options vest and are exercisable each month thereafter, such that options are vested in full on the four-year anniversary of the grant date. During the year ended December 31, 2020, the Company granted stock options under the 2019 Plan that also vest and are exercisable in equal monthly installments over periods of 12 months to 48 months.

On January 1, 2020, the number of the Company's common shares reserved for issuance under the 2019 Plan increased by 980,229 common shares. In addition, 72,186 options forfeited under the 2011 Plan after adoption of the 2019 Plan and became available for issuance under the 2019 Plan. As of December 31, 2020, there were 3,369,348 shares available for issuance under the 2019 Plan, of which 1,663,158 shares were available for future grants.

The total outstanding and exercisable options from the 2011 Plan and 2019 Plan as at December 31 were as follows:

	2020			Weighted average exercise price	2019			Weighted average exercise price
	2019 Plan	2011 Plan	Total		2019 Plan	2011 Plan	Total	
Outstanding at beginning of year - 2011 Plan	—	2,364,526	2,364,526	\$ 2.15	—	2,295,045	2,295,045	\$ 1.77
Outstanding at beginning of year - 2019 Plan	220,140	—	220,140	20.78	—	—	—	—
Granted - 2011 Plan	—	—	—	—	—	116,742	116,742	9.42
Granted - 2019 Plan	1,534,460	—	1,534,460	12.68	287,138	—	287,138	19.95
Exercised - 2011 Plan	—	(226,352)	(226,352)	1.23	—	(33,162)	(33,162)	1.33
Forfeited - 2011 Plan	—	(58,087)	(58,087)	5.94	—	(14,099)	(14,099)	2.66
Forfeited - 2019 Plan	(45,413)	—	(45,413)	18.53	(66,998)	—	(66,998)	17.22
Cancelled - 2019 Plan	(2,997)	—	(2,997)	21.48	—	—	—	—
Outstanding at end of year	<u>1,706,190</u>	<u>2,080,087</u>	<u>3,786,277</u>	<u>\$ 7.29</u>	<u>220,140</u>	<u>2,364,526</u>	<u>2,584,666</u>	<u>\$ 3.75</u>
Outstanding at end of year - Weighted average exercise price	\$ 13.55	\$ 2.15			\$ 20.78	\$ 2.15		
Exercisable at end of year	<u>268,164</u>	<u>1,536,895</u>	<u>1,805,059</u>	<u>\$ 2.94</u>	<u>1,165</u>	<u>1,212,226</u>	<u>1,213,391</u>	<u>\$ 1.65</u>
Exercisable at end of year - Weighted average exercise price	<u>\$ 8.23</u>	<u>\$ 2.02</u>			<u>\$ 17.78</u>	<u>\$ 1.64</u>		

As of December 31, 2020, the weighted average remaining contractual life was 7.86 years [2019 - 7.8 years] for outstanding options. The weighted average remaining contractual life was 6.91 years for vested options [2019 - 7.0 years]. There were 103,500 options forfeited in 2020 [2019 - 81,097] and there were 2,997 options cancelled in 2020 [2019 - nil]. Options granted are valued using the Black-Scholes option pricing model. Amortization of the fair value of the options over vesting years has been expensed and credited to additional paid-in capital in shareholders' deficit. The weighted average fair values of options granted in 2020 was \$8.98 per share [2019 - \$11.81]. Share-based compensation expense recognized for the year ended December 31, 2020 was \$4,945 [2019 - \$1,191].

As of December 31, 2020, there was \$13,012 [2019 - \$6,464] of total unrecognized compensation cost, related to non-vested share options, which is expected to be recognized over a remaining weighted average vesting period of 2.67 years [2019 - 2.60 years].

	2020				2019			
	Number of options			Weighted average fair value	Number of options			Weighted average fair value
	2019 Plan	2011 Plan	Total		2019 Plan	2011 Plan	Total	
Non-vested share options at beginning of year - 2011 Plan	—	1,152,300	1,152,300	\$ 1.88	—	1,706,303	1,706,303	\$ 1.35
Non-vested share options at beginning of year - 2019 Plan	218,975	—	218,975	\$ 14.44	—	—	—	\$ —
Granted - 2011 Plan	—	—	—	—	—	116,742	116,742	6.65
Granted - 2019 Plan	1,534,460	—	1,534,460	8.98	287,138	—	287,138	13.91
Vested, outstanding 2011 Plan	—	(551,026)	(551,026)	1.70	—	(656,646)	(656,646)	1.33
Vested, outstanding 2019 Plan	(269,996)	—	(269,996)	5.80	(1,165)	—	(1,165)	12.16
Forfeited - 2011 Plan	—	(58,082)	(58,082)	4.32	—	(14,099)	(14,099)	1.91
Forfeited - 2019 Plan	(45,413)	—	(45,413)	13.03	(66,998)	—	(66,998)	12.22
Non-vested share options at end of year	<u>1,438,026</u>	<u>543,192</u>	<u>1,981,218</u>	<u>\$ 7.96</u>	<u>218,975</u>	<u>1,152,300</u>	<u>1,371,275</u>	<u>\$ 3.89</u>
Non-vested share options at end of year - Weighted average fair value	<u>\$ 10.28</u>	<u>\$ 1.81</u>			<u>\$ 14.44</u>	<u>\$ 1.88</u>		

The following table summarizes information with respect to share options outstanding as of December 31, 2020:

Exercise price	Options outstanding			Options exercisable		
	Number of options	Weighted average remaining contractual life (years)	Weighted average exercise price	Number of options	Weighted average remaining contractual life (years)	Weighted average exercise price
\$0.84-\$1.00	108,160	2.93	\$ 0.90	108,160	2.93	\$ 0.90
\$1.01-\$2.00	1,219,937	6.58	\$ 1.47	937,898	6.44	\$ 1.44
\$2.01-\$4.00	1,366,657	8.62	\$ 3.22	632,595	8.24	\$ 2.99
\$4.01-\$10.00	147,133	7.67	\$ 8.47	55,701	5.27	\$ 9.28
\$15.01-\$20.00	82,380	8.81	\$ 17.17	24,768	8.77	\$ 17.18
\$21.01-\$22.45	862,010	9.00	\$ 21.65	45,937	8.69	\$ 22.45
Total	<u>3,786,277</u>	<u>7.86</u>	<u>\$ 7.29</u>	<u>1,805,059</u>	<u>6.91</u>	<u>\$ 2.94</u>

The intrinsic value of all outstanding options as of December 31, 2020 was \$11.8 million, based on the fair value of our common shares of \$6.70 per share at December 31, 2020, of which \$7.9 million related to vested options and \$3.9 million related to unvested options.

The fair value of share-based payment transaction is measured using Black-Scholes valuation model. This model also requires assumptions, including expected option life, volatility, risk-free interest rate and dividend yield, which greatly affect the calculated values.

The fair value of options granted was estimated using the Black-Scholes option pricing model, resulting in the following weighted average assumptions for the options granted for the years ended December 31, 2020 and 2019:

	2020	2019
Exercise price	\$ 12.68	\$ 16.90
Share price	\$ 12.68	\$ 16.90
Volatility	85%	80%
Risk-free interest rate	1.03%	1.92%
Expected life	5.88 years	6.21 years
Dividend	0%	0%

Expected volatility is determined using comparable companies for which the information is publicly available. The risk-free interest rate is determined based on the US sovereign rates benchmark in effect at the time of grant with a remaining term equal to the expected life of the option. Expected option life is determined based on the simplified method as the Company does not have sufficient historical exercise data to provide a reasonable basis upon which to estimate expected term. The simplified method is an average of the contractual term of the options and its ordinary vesting period. Dividend yield is based on the share option's exercise price and expected annual dividend rate at the time of grant.

No compensation expense is recorded related to an award for which the transfer to the employee is contingent on the attainment of a performance target until it becomes probable that the performance target will be met.

The Company recognized share-based compensation expense as follows at December 31, 2020 and 2019:

	2020	2019
Administration	\$ 2,007	\$ 574
Research and development	2,055	495
Commercial activities	883	122
	<u>\$ 4,945</u>	<u>\$ 1,191</u>

8 Net loss per share

Basic and diluted net loss per common share is determined by dividing net loss applicable to common shareholders by the weighted average number of common shares and pre-funded warrants outstanding during the period. Share-based compensation shares have been excluded from the calculation because their effects would be anti-dilutive. Therefore, the weighted average number of shares used to calculate both basic and diluted loss per share are the same.

The following potentially dilutive securities have been excluded from the computation of diluted weighted average shares outstanding as of December 31, 2020 and 2019, as they would be anti-dilutive:

	2020	2019
Share options	<u>3,786,277</u>	<u>2,584,666</u>

Amounts in the table above reflect the common share equivalents of the noted instruments.

9 Income taxes

A reconciliation between tax expense and the product of accounting income multiplied by the basic income tax rate for the years ended December 31, 2020 and 2019 is as follows:

	2020	2019
Loss before income taxes	\$ (49,984)	\$ (55,285)
Basic income tax rate	26.33%	26.30%
Computed income tax recovery	(13,161)	(14,542)
Effect on income tax rate resulting from		
Accounting charges not deductible for tax purposes	7	23
Non-deductible share-based compensation	1,310	317
Share issue costs	(984)	(2,739)
Tax benefits of current period losses and other tax assets	12,715	16,829
Valuation allowance for prior year adjustment	108	77
Other	(12)	(21)
Income tax expense recovery reported in the consolidated statements of loss and comprehensive loss	\$ (17)	\$ (56)

The Company has incurred Canadian federal and provincial net operating losses (NOLs) from inception. As of December 31, 2020, the Company has NOL carry-forwards of approximately \$123,494 and \$122,756, respectively, for Canadian federal and Québec purposes, available to reduce future taxable income, which expire beginning in 2027 through 2040. The Company also has scientific research and experimental development expenditures of approximately \$13,735 and \$16,247, respectively, for Canadian federal and Québec income tax purposes, which have not been deducted. These expenditures are available to reduce future taxable income and have an unlimited carry-forward period. Research and development tax credits and expenditures are subject to verification by the tax authorities, and, accordingly, these amounts may vary.

The Company has incurred NOLs for U.S. tax purposes. As of December 31, 2020, the Company has carry-forwards of approximately \$17,428 related to U.S. NOLs that may be carried forward indefinitely and are available to reduce future taxable income.

Deferred income taxes reflect the net tax effects of temporary differences between the carrying amounts of assets and liabilities for financial reporting purposes and the amounts used for income tax purposes. The net deferred tax assets have not been recognized in these financial statements because the criteria for recognition of these assets were not met.

The Company's deferred tax assets consist of the following for the years ended December 31, 2020 and 2019:

	2020	2019
Net operating loss carry-forwards	36,951	25,965
Tax basis of property and equipment in excess of carrying values	100	103
Federal SR&ED investment tax credits	496	535
Taxation of federal SR&ED investment tax credits	(132)	(142)
Research and development expenditures	3,929	2,686
Financing costs	2,583	2,103
Change in tax rates	25	5
Others	34	11
Total gross deferred tax assets	43,986	31,266
Valuation allowance	(43,986)	(31,266)
Net deferred tax assets	—	—

The Company files income tax returns in Canada and in the United States. The Company is subject to Canada Revenue Agency and Revenu Québec examination for fiscal years 2015 to 2020 due to unexpired statute of limitation periods and is subject to US Federal and state income tax examination for fiscal years 2017 to 2020.

10 Government assistance

The Company incurred research and development expenditures that are eligible for investment tax credits. The investment tax credits recorded are based on management's estimates of amounts expected to be recovered and are subject to audit by the taxation authorities. These amounts (expressed in thousands of US dollars) have been recorded as a reduction of research and development expenditures for an amount of \$373 for the year ended December 31, 2020 [2019 - \$392].

11 Commitments

In the normal course of business, the Company enters into contracts with clinical research organizations, drug manufacturers and other vendors for preclinical and clinical research studies, research and development supplies and other services and products for operating purposes. These contracts generally provide for termination on notice, and therefore are cancellable contracts. Therefore, as at December 31, 2020 there are no contractual commitments, except for office leases (note 4).

12 Currency risk

The Company is exposed to the financial risk related to the fluctuation of foreign exchange rates and the degree of volatility of those rates. The foreign currency risk is limited to the portion of the Company's business transactions denominated in currency other than US dollars. The following table provides an indication of the Company's exposure to the Canadian dollar, which is expressed in US dollars as of December 31:

	2020	2019
Cash	\$ 426	\$ 149
Accounts payable and accrued liabilities	675	1,680
Net financial position exposure	<u>\$ 249</u>	<u>\$ 1,531</u>

The Company does not enter into arrangements to hedge its currency risk exposure.

13 Fair value of financial instruments

Pursuant to the accounting guidance for fair value measurement and its subsequent updates, fair value is defined as the price that would be received to sell an asset or paid to transfer a liability (i.e. the exit price) in an orderly transaction between market participants at the measurement date. The accounting guidance establishes a hierarchy for inputs used in measuring fair value that minimizes the use of unobservable inputs by requiring the use of observable market data when available. Observable inputs are inputs that market participants would use in pricing the asset or liability based on active market data. Unobservable inputs are inputs that reflect the assumptions market participants would use in pricing the asset or liability based on the best information available in the circumstances.

The fair value hierarchy is broken down into the three input levels summarized below:

Level 1 — Valuations are based on quoted prices in active markets for identical assets or liabilities and readily accessible by the Company at the reporting date.

Level 2 — Valuations based on inputs other than the quoted prices in active markets that are observable either directly or indirectly in active markets.

Level 3 — Valuations based on unobservable inputs in which there is little or no market data, which requires the Company to develop its own assumptions.

The Company's fair value hierarchy for all its financial assets (by major security type measured at fair value on a recurring basis) for the year ended December 31, 2020, the Company held a Guaranteed investment certificate at Level 1 with a fair value of \$70. For the year ended December 31, 2019 is nil, as there was no financial instruments measured at fair value on a recurring basis as of that date



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

Montreal and Charlotte, N.C., March 29, 2021 -- 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 to 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 COMPREHENSIVE 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

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