Millendo Therapeutics, Inc. Logo

Millendo Therapeutics Reports Full Year 2019 Operating and Financial Results

March 11, 2020

–Topline results from pivotal Phase 2b study of livoletide in patients with Prader-Willi syndrome (PWS) expected in early 2Q20–

–Topline results from first cohort of Phase 2b study of nevanimibe in patients with classic congenital adrenal hyperplasia (CAH) expected in 2H20–

–Successful financings strengthen financial position, extending cash runway into 2022–

ANN ARBOR, Mich.--(BUSINESS WIRE)-- Millendo Therapeutics, Inc. (Nasdaq: MLND), a late-stage biopharmaceutical company primarily focused on developing novel treatments for orphan endocrine diseases, today provided a corporate update and reported financial results for the year ended December 31, 2019.

“In 2019 we achieved critical milestones for our lead asset livoletide, including completing enrollment of the pivotal Phase 2b ZEPHYR study in patients with PWS and building a foundation for our commercial leadership in our new office in Lexington, Massachusetts,” said Julia C. Owens, President and Chief Executive Officer of Millendo Therapeutics. “In addition, we made progress on our Phase 2b study of nevanimibe for patients with CAH, where we are expecting cohort 1 topline data in the second half of 2020, and expanded our pipeline with the addition of MLE-301 for the treatment of menopausal vasomotor symptoms, with first-in-human studies expected to begin in the second half of this year.

“The year 2020 will be an exciting one for Millendo as we continue to advance and build our pipeline of novel treatments for endocrine diseases. With an important topline data readout for livoletide from our pivotal ZEPHYR study in PWS expected in early second quarter, we look forward to building an integrated commercial organization to support future plans for what could be the first pharmacological treatment to address hyperphagia in patients with PWS.”

Fourth Quarter 2019 and Recent Highlights

- **3-month core period of the pivotal Phase 2b study of livoletide in patients with PWS has been completed:** 158 patients were randomized across 38 clinical trial sites worldwide, with an average baseline HQ-CT score of approximately 20. A total of 156 patients completed the 3-month core period with 2 subjects (~1%) discontinuing; all 156 patients have moved into the nine-month extension. The company expects to report topline data from the pivotal Phase 2b ZEPHYR study in early 2Q20. The Phase 2b study has the potential to support a New Drug Application (NDA) for livoletide.
- **Continued to advance Phase 2b clinical study of nevanimibe in patients with CAH:** The Phase 2b study is ongoing with two separate cohorts of patients. Topline data from the first cohort of the study is expected in 2H20 and includes patients with elevated levels of 17-hydroxyprogesterone (17-OHP) greater than or equal to 4-times the upper limit of normal (ULN). The second cohort is continuing enrollment and includes patients with high exogenous cortisol doses and a baseline 17-OHP level of less than 4-times the ULN.
- **New pipeline asset MLE-301 expected to enter clinical trials in 2H20:** MLE-301, a selective neurokinin 3 receptor (NK3R) antagonist, is intended for the treatment of vasomotor symptoms (VMS), commonly known as hot flashes and night sweats, in menopausal women. MLE-301 is currently in preclinical studies designed to enable first-in-human clinical studies, which the company expects to initiate in the second half of 2020.
- **Strengthened Company Leadership:** Christophe Arbet-Engels, MD, PhD, was appointed Chief Medical Officer in February 2020, building upon the expanded Millendo leadership team including the appointments of Thomas Hoover as Chief Commercial Officer, Tamara Joseph as General Counsel and Ryan Zeidan, PhD, as Chief Development Officer. In addition, Geoff Nichol, MB, ChB, MBA, Chief Medical Officer of BioMarin, joined Millendo’s board of directors.

Anticipated 2020 Milestones

- Report topline data from the pivotal Phase 2b study of livoletide in patients with PWS early in the second quarter of 2020.
- Report topline data from the first cohort of the Phase 2b study of nevanimibe in patients with CAH in the second half of 2020.
- Begin first-in-human studies of MLE-301 in the second half of 2020.

“With an additional $34.7 million in gross proceeds from our successful financing in December 2019 as well as sales under our ATM facility, we are pleased that our current cash position is expected to support our capital needs into 2022,” commented Louis J. Arcudi III, Chief Financial Officer.

Full Year 2019 Financial Results

Cash Position: Cash, cash equivalents, marketable securities and restricted cash were $63.5 million at December 31, 2019, compared to $78.2 million at December 31, 2018.

Research and Development (R&D) Expenses: R&D expenses were $27.8 million for 2019, as compared to $14.4 million for the same period in 2018. The increase in R&D expenses was primarily driven by support of the company’s Phase 2b clinical study of livoletide and increased headcount.

General and Administrative (G&A) Expenses: G&A expenses were $17.6 million for 2019, as compared to $8.7 million for the same period in 2018.

The increase in G&A expenses was primarily driven by increased costs related to employee compensation and professional fees to support ongoing business operations and compliance with obligations associated with being a publicly traded company.

Net Loss: The company's net loss for the year ended December 31, 2019 was $44.6 million compared to $27.2 million for the year ended December 31, 2018.

2020 Financial Guidance
Millendo expects that its cash, cash equivalents, marketable securities and restricted cash will support the company's capital needs into 2022. This cash runway guidance is based on the company's current operational plans and excludes any additional funding that may be received or business development or commercialization activities that may be undertaken.

About Livoletide
Livoletide is an unacylated ghrelin analogue in late-stage clinical development for the treatment of hyperphagia in Prader-Willi syndrome (PWS). This rare genetic disease is characterized by hyperphagia, a chronic unrelenting hunger, that leads to obesity, metabolic dysfunction, reduced quality of life and early mortality. Livoletide may provide the first treatment for hyperphagia in patients with PWS by addressing the underlying hormone dysregulation that contributes to the disease. In a previous randomized, double-blind, placebo-controlled Phase 2a clinical trial in 47 patients with PWS, administration of livoletide once daily for two weeks was associated with a clinically meaningful improvement in hyperphagia, as well as a reduction in appetite. Millendo has received both Orphan Drug Designation and Fast Track Designation for livoletide for the treatment of hyperphagia in PWS from the U.S. Food and Drug Administration (FDA) and Orphan Drug Designation from the European Medicines Agency (EMA). For more information about Millendo's pivotal study of livoletide (ZEPHYR) please visit www.clinicaltrials.gov (NCT03790865) or the Patients and Families portion of our website.

About the ZEPHYR study
The ZEPHYR study is a two-part, randomized, double-blind, placebo-controlled pivotal Phase 2b/3 study. The first part is a pivotal Phase 2b study that includes a three-month double-blind, placebo-controlled core period in which patients receive one of two doses of livoletide or placebo followed by a nine-month extension period in which all patients receive livoletide. The Phase 2b study enrolled 158 patients across 38 clinical sites in the United States, Europe and Australia. The second part is a Phase 3 study that will consist of a six-month double-blind, placebo-controlled core period in which patients will receive livoletide or placebo followed by a six-month extension period in which all patients receive livoletide. The study's primary endpoint measures the change in food-related behaviors using the validated Hyperphagia Questionnaire for Clinical Trials (HQ-CT) during each core period. ZEPHYR is a pivotal study and the results of the Phase 2b study may be sufficient to support a new drug application (NDA) for livoletide.

About Nevanimibe
Nevanimibe decreases adrenal steroidogenesis through the inhibition of acyl coenzyme A: cholesterol acyltransferase 1, or ACAT1, and is being studied for the treatment of classic congenital adrenal hyperplasia (CAH). CAH is a rare, monogenic adrenal disease that requires lifelong treatment with exogenous cortisol, often at high doses. These chronic high doses of cortisol can result in side effects that include diabetes, obesity, hypertension and psychological problems. Millendo has received Orphan Drug Designation for nevanimibe for the treatment of CAH from the FDA and the EMA. In a Phase 2a clinical trial in patients with CAH, Millendo observed nevanimibe to be associated with clear signs of clinical activity in seven of 10 treated patients. A Phase 2b trial of nevanimibe in CAH (NCT03669549) is ongoing.

About MLE-301
MLE-301 is a neurokinin 3 receptor (NK3R) antagonist that is being developed as a potential treatment of VMS, commonly known as hot flashes and night sweats, in menopausal women. NK3R plays a key role in regulating the activity of KNDy (kisspeptin/NKB/dynorphin) neurons, which are believed to participate in the generation of VMS. By inhibiting the NK3R signaling on the KNDy neurons and potentially other NK3R-expressing neurons that propagate heat dissipation signals through the hypothalamus, MLE-301 aims to reduce the effects of hyperactive KNDy neurons and thereby address the excessive heat dissipation signaling associated with VMS. MLE-301 is currently in preclinical studies designed to enable first-in-human clinical studies.

About Millendo Therapeutics, Inc.
Millendo Therapeutics is a late-stage biopharmaceutical company primarily focused on developing novel treatments for orphan endocrine diseases where current therapies do not exist or are insufficient. Millendo seeks to create distinct and transformative treatments where there is a significant unmet medical need. The company is currently advancing livoletide for the treatment of hyperphagia in Prader-Willi syndrome, nevanimibe for the treatment of classic congenital adrenal hyperplasia and MLE-301 for the treatment of vasomotor symptoms associated with menopause. For more information, please visit www.millendo.com.

Cautionary Statement Regarding Forward-Looking Statements
Certain statements contained in this press release regarding matters that are not historical facts, are forward-looking statements within the meaning of Section 27A of the Securities Act of 1933, as amended and Section 21E of the Securities Exchange Act of 1934, as amended. These include statements regarding our plans to develop and commercialize our product candidates and the progress and timing of our ongoing and planned clinical trials for our product candidates and the sufficiency of our current capital resources, and, therefore, you are cautioned not to place undue reliance on them. In some cases, you can identify forward-looking statements by the words "may," "might," "will," "could," "would," "should," "expect," "intend," "plan," "objective," "anticipate," "believe," "estimate," "predict," "project," "potential," "continue" and "ongoing," or the negative of these terms, or other comparable terminology intended to identify statements about the future. Such forward-looking statements are based on Millendo's expectations and involve risks and uncertainties; consequently, actual results may differ materially from those expressed or implied in the statements due to a number of factors, including that Millendo has incurred significant losses since inception, Millendo has a limited operating history and has never generated any revenue from product sales, Millendo will require additional capital to finance its operations, Millendo's future success is dependent on the successful clinical development, regulatory approval and subsequent commercialization of livoletide, nevanimibe and any future product candidates, preclinical studies or earlier clinical trials are not necessarily predictive of future results and the results of Millendo's clinical trials may not support Millendo's livoletide or nevanimibe claims, Millendo may encounter substantial delays in its clinical trials or Millendo may fail to demonstrate safety and efficacy to the satisfaction of applicable regulatory authorities, enrollment and retention of patients in clinical trials is an expensive and time-consuming process and could be made more difficult or rendered impossible by multiple factors outside Millendo's control, Millendo's product candidates may cause undesirable side effects or have other properties that could delay or prevent their regulatory approval, or limit their commercial potential and Millendo faces substantial competition. You should refer to the risk factor disclosure set forth in the periodic reports and other documents we file with the Securities and Exchange Commission available at www.sec.gov, including without limitation, in the section entitled "Risk Factors" in our Annual Report on Form 10-K for our fiscal year ended December 31, 2019 and subsequent reports that we file with the Securities and Exchange Commission

including our Annual Report on Form 10-K for the fiscal year ended December 31, 2019.

New factors emerge from time to time and it is not possible for Millendo to predict all such factors, nor can Millendo assess the impact of each such factor on the business or the extent to which any factor, or combination of factors, may cause actual results to differ materially from those contained in any forward-looking statements. Forward-looking statements included in this press release are based on information available to Millendo as of the date of this press release. Millendo disclaims any obligation to update such forward-looking statements to reflect events or circumstances after the date of this press release, except as required by applicable law.

Millendo Therapeutics, Inc.
Condensed Statements of Operations
(in thousands except share and per share amounts)

	Year Ended December 31,	
	2019	**2018**
Operating Expenses		
Research and development	$ 27,843	$ 14,425
General and administrative	17,556	8,691
Other general expenses	-	3,758
Loss from operations	45,399	26,874
Other (income) expense, net	(831)	303
Net loss	(44,568)	(27,177)
Net loss attributable to noncontrolling interest	-	(15)
Net loss attributable to common stockholders	$ (44,568)	$ (27,192)
Net loss per share of common stock, basic and diluted	$ (3.25)	$ (17.58)
Weighted-average shares of common stock outstanding, basic and diluted	13,706,744	1,547,051

Millendo Therapeutics, Inc.
Condensed Balance Sheet Data
(in thousands)

	December 31,	December 31,
	2019	**2018**
Cash, cash equivalents, marketable securities and restricted cash	$ 63,512	$ 78,155
Other assets	11,458	5,919
Total assets	$ 74,970	$ 84,074
Total liabilities	$ 15,099	$ 10,952
Total stockholders' equity	59,871	73,122

Total liabilities and stockholders' equity	$ 74,970	$ 84,074

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