Millendo Therapeutics, Inc. Logo

Millendo Therapeutics Reports Third Quarter 2019 Operating and Financial Results

November 13, 2019

– Livoletide pivotal study in Prader-Willi syndrome (PWS) completed recruitment for patients ages 8 to 65 and is on track to report topline results in 1H20 –

– Company to host PWS Breakfast Symposium on Thursday, November 14, 2019, to discuss potential new treatment paradigm for PWS patients with livoletide –

– Nevanimibe Phase 2b study in congenital adrenal hyperplasia (CAH) continues recruitment, with topline results expected for cohort 1 in 2H20 –

ANN ARBOR, Mich.--(BUSINESS WIRE)--Nov. 13, 2019-- Millendo Therapeutics, Inc. (Nasdaq: MLND), a clinical-stage biopharmaceutical company primarily focused on developing novel treatments for orphan endocrine diseases with significant unmet needs, today provided a corporate update and reported financial results for the quarter ended September 30, 2019.

"With the Phase 2b portion of our ZEPHYR pivotal Phase 2b/3 clinical study of livoletide in PWS patients now fully recruited for patients ages 8 to 65, we look forward to reporting topline results in the first half of 2020," said Julia C. Owens, President and Chief Executive Officer of Millendo Therapeutics. "We are excited about the potential for livoletide to treat hyperphagia and other food-seeking behaviors that lead to morbidity and mortality in these patients, as well as significant caregiver burden.

"In addition, our Phase 2b clinical study of nevanimibe in CAH is continuing recruitment across its two cohorts, providing us with further insights on the activity of nevanimibe over longer treatment periods and in CAH patients where steroid doses are reduced. We expect to report topline results from the study's first cohort in the second half of 2020."

Third Quarter 2019 and Recent Highlights

- **Patient recruitment completed for the Phase 2b portion of ZEPHYR for patients ages 8 to 65, eight months after study initiation, with topline results expected in 1H20**. This study, one of the largest PWS studies ever conducted with over 150 patients recruited across 38 clinical trial sites worldwide, is evaluating the safety and efficacy of livoletide on hyperphagia and food-related behaviors in patients with Prader-Willi syndrome. The study's primary endpoint measures the change in hyperphagia and other food-related behaviors using the validated Hyperphagia Questionnaire for Clinical Trials (HQ-CT). Secondary outcome measures include body weight, fat mass and waist circumference. Millendo continues to implement a protocol amendment globally adding an additional cohort of PWS patients ages 4 to 7. Sites that recruit pediatric patients will continue to actively recruit patients for this cohort.

- **Millendo is hosting a PWS breakfast symposium on November 14 in New York City.** Shawn McCandless, M.D., Professor and Head of Section, Genetics and Metabolism - Department of Pediatrics, University of Colorado School of Medicine and Children's Hospital of Colorado and company management will discuss the epidemiology of the disease, the current treatment landscape and the opportunity for livoletide.

- **Topline results from the ongoing Phase 2b study of nevanimibe in patients with CAH are anticipated for cohort 1 in 2H20.** The Phase 2b study will evaluate nevanimibe's efficacy and safety in treating CAH and includes two distinct cohorts of patients. Cohort 1 includes patients with a baseline 17-hydroxyprogesterone (17-OHP) level, a key measure of disease control, of greater than or equal to 4-times the upper limit of normal (ULN) which is similar to the Phase 2a study population but now with longer duration of treatment and with the ability for additional dose-ranging. Cohort 2 includes patients with elevated glucocorticoids and a baseline 17-OHP level of less than 4-times the ULN. Patients in cohort 2 will first have their glucocorticoid dose reduced, with an anticipated increase in 17-OHP levels and then treatment with nevanimibe seeking to reduce the 17-OHP levels. The study's primary endpoint across both study cohorts is an assessment of the percentage of patients that achieve 17-OHP levels of less than or equal to 2-times the ULN. Secondary endpoints include assessments of other adrenal hormones, including androgens.

Third Quarter 2019 Financial Results

Cash Position: Cash, cash equivalents, marketable securities and restricted cash were $48.3 million at September 30, 2019, compared to $78.2 million at December 31, 2018.

Research and Development (R&D) Expenses: R&D expenses were $7.3 million for the third quarter of 2019, as compared to $3.9 million for the same period in 2018. The increase in R&D expenses was primarily driven by increased spending on the company's pivotal Phase 2b/3 study of livoletide in PWS and higher compensation expenses as a result of increased headcount.

General and Administrative (G&A) Expenses: G&A expenses were $4.4 million for the third quarter of 2019, as compared to $3.9 million for the same period in 2018. The increase in G&A expenses was primarily driven by higher compensation expense as a result of increased headcount and increased expenses to support ongoing business operations and obligations associated with being a publicly traded company.

Net Loss: The company's net loss for the quarter ended September 30, 2019 was $11.6 million as compared to $7.9 million for the same period in 2018.

2019 Financial Guidance

Millendo expects that its cash, cash equivalents, marketable securities and restricted cash will support the company's capital needs through the fourth quarter of 2020, beyond the readout for the topline results of the Phase 2b portion of the pivotal Phase 2b/3 study of livoletide in PWS, which is expected in the first half of 2020. This cash runway guidance is based on the company's current operational plans and excludes any additional funding or business development activities.

About Livoletide
Livoletide is an unacylated ghrelin analogue in late-stage clinical development for the treatment of Prader-Willi syndrome (PWS), a rare genetic disease characterized by hyperphagia, a chronic unrelenting hunger, that leads to obesity, metabolic dysfunction, reduced quality of life and early mortality. In a previous randomized, double-blind, placebo-controlled Phase 2 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 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 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 portion of the study, one of the largest global PWS studies ever conducted, has completed recruitment for patients ages 8 to 65 with over 150 patients across 38 clinical sites in the United States, Europe and Australia. Millendo continues to implement a protocol amendment globally adding an additional cohort of PWS patients ages four to seven. Sites that recruit pediatric patients will continue to actively recruit patients for this cohort. 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 portion of the study may be sufficient to support a new drug application (NDA) for livoletide.

About Nevanimibe
Nevanimibe decreases adrenal steroidogenesis through the inhibition of 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, which can make it difficult for physicians to appropriately treat CAH without causing adverse consequences. In a Phase 2 proof-of-concept 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 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. As a leading orphan endocrine company, Millendo creates distinct and transformative treatments where there is a significant unmet medical need. The company is currently advancing livoletide for the treatment of 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 Millendo's expectations regarding the potential for an NDA submission for livoletide, the timing for its clinical trials, including the timing of topline data from the Phase 2b portion of its ZEPHYR trial and timing of topline data from the first cohort from the Phase 2b clinical study of nevanimibe in CAH, and Millendo's expectations regarding its cash runway, 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 our Quarterly Report on Form 10-Q for our fiscal quarter ended September 30, 2019 and subsequent reports that we file with the Securities and Exchange Commission.

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)

	Three Months Ended September 30,		Nine Months Ended September 30,	
	2019	**2018**	**2019**	**2018**
Operating Expenses				
Research and development	$ 7,308	$ 3,871	$ 19,493	$ 9,840
General and administrative	4,443	3,935	13,075	7,340
Loss from operations	11,751	7,806	32,568	17,180
Other (income) expense, net	(119)	85	(699)	139
Net loss	(11,632)	(7,891)	(31,869)	(17,319)
Net loss attributable to noncontrolling interest	-	355	-	676
Net loss attributable to common stockholders	$ (11,632)	$ (7,536)	$ (31,869)	$ (16,643)
Net loss per share of common stock, basic and diluted	$ (0.87)	$ (10.61)	$ (2.38)	$ (23.43)
Weighted-average shares of common stock outstanding, basic and diluted	13,420,614	710,390	13,386,381	710,390

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

	September 30,	December 31,
	2019	**2018**
Cash, cash equivalents, marketable securities and restricted cash	$ 48,348	$ 78,155
Other assets	10,131	5,919
Total assets	$ 58,479	$ 84,074
Total liabilities	$ 13,650	$ 10,952
Total stockholders' equity	44,829	73,122
Total liabilities and stockholders' equity	$ 58,479	$ 84,074

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