Millendo Therapeutics, Inc. Logo

Millendo Therapeutics Announces Topline Results for Pivotal Phase 2b Study of Livoletide in Patients with Prader-Willi Syndrome (PWS)

April 6, 2020

– Livoletide did not achieve statistically significant improvement in primary endpoint of change in hyperphagia and food-related behaviors relative to placebo –

– Millendo to discontinue livoletide program in PWS and focus on development of pipeline assets nevanimibe and MLE-301 –

– Company to host a conference call today at 8:15 a.m. EDT –

ANN ARBOR, Mich.--(BUSINESS WIRE)--Apr. 6, 2020-- Millendo Therapeutics, Inc. (Nasdaq: MLND), a biopharmaceutical company primarily focused on developing novel treatments for endocrine diseases, announced today that it is discontinuing the development of livoletide as a potential treatment for Prader-Willi syndrome (PWS). The decision to discontinue the PWS program was based on topline data from the pivotal Phase 2b ZEPHYR study which showed that treatment with livoletide did not result in a statistically significant improvement in hyperphagia and food-related behaviors as measured by the Hyperphagia Questionnaire for Clinical Trials (HQ-CT) compared to placebo.

"Unfortunately, treatment with livoletide did not significantly improve hyperphagia and food-related behaviors in our ZEPHYR study. While we are disappointed in these results, I want to recognize our team's hard work and commitment in executing this robust study that informed the difficult decision to discontinue the livoletide PWS program," said Julia C. Owens, President and Chief Executive Officer of Millendo Therapeutics. "We are deeply grateful to the patients, caregivers and researchers who made the ZEPHYR study possible. We are committed to understanding the totality of the Phase 2b results and we intend to report the data at a future scientific meeting or publication when they are available."

Owens added, "Moving forward, we will shift our development focus to compelling portfolio programs nevanimibe for congenital adrenal hyperplasia (CAH) and MLE-301 for menopausal vasomotor symptoms. With the rapidly evolving COVID-19 global pandemic and the extraordinary burden it has put on hospitals and healthcare providers, we are monitoring the potential impact of the situation on these programs and will provide an update when we have more clarity on expected timelines."

The ZEPHYR study was a two-part, randomized, double-blind, placebo-controlled pivotal Phase 2b/3 study. The pivotal Phase 2b study included a three-month double-blind, placebo-controlled period in which patients (N=158) were randomized to either 60 µg/kg or 120 µg/kg of livoletide, or placebo. The Phase 2b data showed improvements from baseline in HQ-CT scores of -4.7 (p = 0.13) and -3.8 (p = 0.45) for the livoletide treated groups (60 µg/kg or 120 µg/kg, respectively) at 12 weeks compared to -2.8 for placebo. The average HQ-CT baseline score was 20.2. No positive trends were observed for any of the secondary endpoints of fat mass, body weight or waist circumference.

Livoletide was well tolerated during the ZEPHYR study, with injection site reaction being the most frequently reported adverse event, as expected with an injectable drug, and mostly mild in severity. A total of 2 patients (1.3%) dropped out of the study during the 12-week core period. There were 4 serious adverse events reported during the 12-week period, with none being related to livoletide treatment.

Millendo has made the decision to stop all livoletide development efforts in PWS, including the 9-month extension study and initiation of the Phase 3 ZEPHYR study.

Conference Call Information
The company will host a live conference call and webcast today at 8:15 a.m. EDT to discuss the topline results. The conference call may be accessed by dialing (866) 524-3160 (domestic) or +1 (412) 317-6760 (international). A live webcast of the conference call will be available on the Investors & Media section of Millendo's website at http://investors.millendo.com. A replay of the webcast will be archived on Millendo's website for 30 days following the presentation.

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. In a randomized, double-blind, placebo-controlled pivotal Phase 2b clinical trial in 158 patients with PWS, administration of livoletide once daily for 12 weeks showed that livoletide did not result in a statistically significant improvement in hyperphagia and food-related behaviors. For more information about Millendo's pivotal study of livoletide (ZEPHYR) please visit www.clinicaltrials.gov (NCT03790865) or the Our Patients portion of our website.

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 U.S. Food and Drug Administration (FDA) and the European Medicines Agency (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 biopharmaceutical company primarily focused on developing novel treatments for 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 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. 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. These include statements with respect to Millendo's plans to provide a further update on the results of the ZEPHYR study and the timelines for nevanimibe and MLE-301 and the continued development of nevanimibe for congenital adrenal hyperplasia and MLE-301 for menopausal vasomotor symptoms, and, therefore, you are cautioned not to place undue reliance on them. 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 current 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 product candidate 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, Millendo faces substantial competition and Millendo's business, preclinical studies and clinical development programs and timelines, its financial condition and results of operations could be materially and adversely affected by the current COVID-19 pandemic. You should refer to the risk factor disclosure set forth in the periodic reports and other documents Millendo files with the Securities and Exchange Commission available at www.sec.gov, including without limitation in the section entitled "Risk Factors" in Millendo's Annual Report on Form 10-K for the fiscal year ended December 31, 2019, Millendo's Current Report on Form 8-K filed on April 6, 2020 and subsequent current and periodic reports that Millendo files 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.

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