Millendo Therapeutics, Inc. Logo

Millendo Therapeutics Completes Patient Recruitment for Pivotal Study of Livoletide in Prader-Willi Syndrome

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– ZEPHYR study successfully recruits over 150 patients in eight months for Phase 2b portion of pivotal Phase 2b/3 study of livoletide in PWS patients ages 8 to 65 –

– Recruitment will continue for a separate cohort of PWS patients ages 4 to 7 –

ANN ARBOR, Mich.--(BUSINESS WIRE)--Nov. 13, 2019-- Millendo Therapeutics, Inc. (Nasdaq: MLND), a clinical-stage biopharmaceutical company developing novel treatments primarily for orphan endocrine diseases, today announced that the Phase 2b portion of its pivotal Phase 2b/3 clinical study of livoletide in patients with Prader-Willi syndrome (PWS), called ZEPHYR, has completed recruitment for patients ages 8 to 65. The 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 food-related behaviors in patients with PWS. The primary endpoint of the study is an assessment of livoletide's impact on hyperphagia, the constant feeling of excessive hunger that is a hallmark symptom of the disease. Topline results are expected in the first half of 2020 and may support a New Drug Application (NDA) filing for livoletide.

"Completing recruitment of over 150 patients in this pivotal study is an important step forward as we continue our efforts to advance livoletide for PWS patients and families who struggle with the life-threatening symptoms of the disease, especially hyperphagia," said Julia C. Owens, President and Chief Executive Officer of Millendo Therapeutics. "This achievement is also an important milestone for Millendo. Ahead of sharing topline results from the Phase 2b portion of ZEPHYR in the first half of next year, we are preparing for a build of our commercial organization in the Boston area."

The ZEPHYR study, initiated in March 2019, is a two-part, randomized, double-blind, placebo-controlled pivotal Phase 2b/3 study. The Phase 2b portion 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. All patients who have completed the core period of the Phase 2b portion of the study thus far have entered into the nine-month safety extension period. 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 these patients.

The second part of ZEPHYR is a Phase 3 study that will recruit additional patients at the same clinical sites and consists 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 primary endpoint for both the Phase 2b and Phase 3 portions of ZEPHYR is the change in food-related behaviors using the validated Hyperphagia Questionnaire for Clinical Trials (HQ-CT).

"We are very pleased with how quickly our participating trial sites were able to recruit for ZEPHYR. Throughout this process, we have seen enthusiasm from our active sites and we appreciate their partnership as we work toward our common goal of addressing hyperphagia in PWS," said Ryan Zeidan, Chief Development Officer of Millendo Therapeutics. "We are grateful to the patients, families and caregivers and investigators involved in the study for their commitment and dedication."

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 Prader-Willi Syndrome
Prader-Willi syndrome (PWS) is a genetic disease caused by the lack of expression of several genes on chromosome 15, which leads to hyperphagia, intellectual disability, short stature and incomplete sexual development, among other symptoms. PWS patients are at risk of premature mortality, mainly from obesity related conditions such as cardiovascular disease, respiratory distress and from accidents. The incidence of PWS is approximately 1 in 15,000 births. The prevalence of PWS is estimated between 8,000-11,000 patients in the United States and 13,000-18,000 in Europe. Currently, there is no effective or approved treatment for hyperphagia and abnormal eating behaviors associated with PWS. Growth hormone is used for improvement in height, cognition and body composition, but has no effect on appetite and over-eating. The only way to effectively manage hyperphagia, obesity and related complications of PWS is strict control over access to food, creating significant burden for families and caregivers.

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 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 timing of data from its clinical trials, including the timing of topline data from the Phase 2b portion of its ZEPHYR trial, and the potential for an NDA submission for livoletide, 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 our including in the section entitled "Risk Factors" in our Quarterly Report on Form 10-Q for our fiscal quarter ended June 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.

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Source: Millendo Therapeutics, Inc.

Millendo Investor Contact:
Stephanie Ascher
Stern Investor Relations
212-362-1200
stephanie.ascher@sternir.com

Millendo Media Contact:
Julie Bane
MacDougall
617-821-1089
jbane@macbiocom.com