Millendo Therapeutics Initiates Pivotal Phase 2b/3 Clinical Study of Livoletide for the Treatment of Prader-Willi Syndrome

Top-line Phase 2b results are expected in 1H 2020 and may support an NDA submission for livoletide

ANN ARBOR, Mich., March 19, 2019 – Millendo Therapeutics, Inc. (Nasdaq: MLND), a clinical-stage biopharmaceutical company developing novel treatments for orphan endocrine diseases, announced today that it has initiated its pivotal Phase 2b/3 clinical study investigating livoletide (AZP-531) in patients with Prader-Willi syndrome (PWS). The study, called ZEPHYR, will evaluate the safety and efficacy of livoletide on food-related behaviors in PWS patients. The primary endpoint is an assessment of livoletide's impact on hyperphagia, the excessive hunger which is a hallmark symptom of the disease. ZEPHYR is one of the largest global PWS studies ever conducted and its Phase 2b portion aims to recruit 150 patients from up to 40 clinical sites in the United States and Europe.

Maithé Tauber, M.D., Professor of Pediatrics at the University of Toulouse and Chief of Endocrinology and Medical Genetics at the Children's Hospital of Toulouse noted, "Livoletide is an exciting investigational drug for Prader-Willi syndrome that has the potential to treat hyperphagia, the unrelenting hunger that often leads to excessive eating and is a root cause of morbidity and mortality in PWS patients. Current strategies to manage the disease present a heavy burden on caregivers, and do not address the underlying hyperphagia experienced by patients. Livoletide has the potential to be an important new treatment option that could positively impact the lives of patients and their caregivers."

The ZEPHYR study is a two-part, randomized, double-blind, placebo-controlled pivotal Phase 2b/3 study. The study will begin with a Phase 2b portion 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 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). ZEPHYR is a pivotal study and the results of the Phase 2b portion of the Phase 2b/3 study may support a new drug application (NDA) for livoletide.

Pharis Mohideen, M.D., Chief Medical Officer at Millendo, further noted, "The initiation of our pivotal trial for livoletide is an important step toward our mission to bring life-changing therapies to market for rare endocrine diseases with significant unmet medical needs, like Prader-Willi syndrome. We are pleased that multiple clinical sites are actively enrolling patients, and we expect to report topline results from the Phase 2b portion of ZEPHYR in the first half of 2020."

Additional details about the ZEPHYR Study can be found at www.clinicaltrials.gov, NCT03790865 and millendo.com/patients-and-families.

About Livoletide
Millendo's lead asset, 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 randomized, double-blind, placebo-controlled Phase 2 clinical trial in 47 patients with PWS, administration of livoletide once daily was associated with a clinically meaningful improvement in hyperphagia, as well as a reduction in appetite. Millendo has received orphan drug designation for livoletide from the U.S. Food and Drug Administration, or FDA, and the European Medicines Agency, or EMA, for the treatment of PWS. For more information about Millendo Therapeutics' pivotal study of livoletide (ZEPHYR) please visit www.clinicaltrials.gov (NCT03790865).

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 intellectual disability, short stature, incomplete sexual development and hyperphagia, among other symptoms. PWS patients are at risk of premature mortality, usually by the age of 30-40, 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 Millendo Therapeutics, Inc.
Millendo Therapeutics is a late-stage biopharmaceutical company 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 and nevanimibe for the treatment of classic congenital adrenal hyperplasia

and endogenous Cushing's syndrome. 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 21E of the Securities Exchange Act of 1934, as amended, and the Private Securities Litigation Reform Act of 1995, known as the PSLRA. These include statements regarding management's intentions, plans, beliefs, expectations or forecasts for the future, and, therefore, you are cautioned not to place undue reliance on them. No forward-looking statement can be guaranteed, and actual results may differ materially from those projected. Millendo undertakes no obligation to publicly update any forward-looking statement, whether as a result of new information, future events or otherwise, except to the extent required by law. Millendo uses words such as "anticipates," "believes," "plans," "expects," "projects," "future," "intends," "may," "will," "should," "could," "estimates," "predicts," "potential," "continue," "guidance," and similar expressions to identify these forward-looking statements that are intended to be covered by the safe-harbor provisions of the PSLRA. 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 Millendo's plans to develop and commercialize its product candidates, including livoletide and nevanimibe; the timing of initiation of Millendo's planned clinical trials; the timing of the availability of data from Millendo's clinical trials; the timing of any planned investigational new drug application or new drug application; Millendo's plans to research, develop and commercialize its current and future product candidates; the clinical utility, potential benefits and market acceptance of Millendo's product candidates.

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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