Millendo Therapeutics Announces Initiation of Phase 2b Clinical Trial of Nevanimibe in Patients with Classic Congenital Adrenal Hyperplasia

ANN ARBOR, Mich., September 13, 2018 – Millendo Therapeutics, Inc., a clinical-stage biopharmaceutical company focused on developing novel treatments for orphan endocrine diseases, today announced the initiation of a Phase 2b clinical trial evaluating the efficacy and safety of its novel drug candidate, nevanimibe, in patients with classic congenital adrenal hyperplasia (CAH), a rare inherited endocrine disease characterized by abnormal hormone levels and overgrowth of the adrenal glands.

"The advancement of nevanimibe into a Phase 2b trial marks a significant milestone for Millendo as we work to deliver meaningful therapies to patients who do not have sufficient treatment options," said Julia Owens, Ph.D., President and Chief Executive Officer. "We are encouraged by the positive Phase 2 efficacy and safety results of nevanimibe that were presented at the Endocrine Society's annual meeting in March, in which these data demonstrated reductions in key steroids and steroid precursors including promising reductions in 17-OHP, a key measure of disease control. Our Phase 2b trial will explore longer durations of treatment and higher doses of nevanimibe with the objective of enabling more patients to achieve hormonal control."

The open-label, intra-subject dose-escalation trial will enroll a total of 20 to 24 adult CAH patients who are currently taking glucocorticoids, the current standard of care. Patients will receive nevanimibe for a total of 12 consecutive weeks starting at a dose of 1,000 mg BID. Dose escalation to 1,500 mg BID or 2,000 mg BID will be based on 17-hydroxyprogesterone (17-OHP) levels. The primary endpoint will be an assessment of the percentage of patients that achieve 17-OHP levels less than or equal to two times the upper limit of normal (ULN). Secondary endpoints include assessments of other adrenal hormones, including androgens. For additional information on this clinical trial, please visit www.clinicaltrials.gov and reference identifier number NCT03669549.

About Classic Congenital Adrenal Hyperplasia

Classic congenital adrenal hyperplasia (CAH) is a rare inherited endocrine disease caused by a genetic mutation preventing cortisol synthesis and is characterized by overgrowth of the adrenal glands, adrenal insufficiency, mineralocorticoid deficiency, and androgen excess. Classic CAH has a prevalence of 15-18,000 patients in the U.S. and about 40,000 patients in Europe. The most frequent cause of CAH, responsible for 95% of cases, is a deficiency in the enzyme 21-hydroxylase, which is required for the production of cortisol and aldosterone in the adrenal glands. Classic CAH is diagnosed at birth as part of universal screening. It can lead to severe virilization in women, testicular tumors in men, and infertility.

About Nevanimibe

Nevanimibe (formerly ATR-101) is an adrenal-selective inhibitor of ACAT1 (acyl-CoA:cholesterol acyltransferase 1), an enzyme that catalyzes the transformation of free cholesterol into cholesterol ester, the starting point for adrenal steroid synthesis. In preclinical studies, nevanimibe was observed to be associated with dose and time-dependent decreases in levels of adrenal steroids and steroid precursors, including those particularly elevated in CAH. Nevanimibe is also in Phase 2 development for Cushing's syndrome (NCT03053271).

About Millendo Therapeutics, Inc.
Millendo Therapeutics is focused on developing novel treatments for orphan endocrine diseases. The Company's objective is to build a leading endocrine company that creates distinct and transformative treatments for a wide range of diseases 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.

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