



Millendo Therapeutics Announces Initiation of Phase 2 Clinical Trial of ATR-101 in Patients with Endogenous Cushing's Syndrome

ANN ARBOR, Mich., Mar. 28, 2017 – [Millendo Therapeutics, Inc.](#), a clinical-stage biopharmaceutical company focused on developing novel treatments for endocrine diseases caused by hormone dysregulation, today announced the initiation of a Phase 2 clinical trial evaluating the safety and efficacy of a novel oral drug candidate, ATR-101, in patients with endogenous Cushing's syndrome (CS), a rare endocrine disease characterized by increased cortisol production from the adrenal glands. ATR-101, an adrenal-selective small molecule inhibitor of ACAT1, is also being studied in an ongoing Phase 2 clinical trial in patients with classic congenital adrenal hyperplasia (CAH) and a Phase 1 clinical trial in patients with adrenocortical carcinoma (ACC).

"The initiation of this Phase 2 clinical trial of ATR-101 in endogenous CS marks an important milestone in the development of this product candidate in endocrine diseases where current therapies are insufficient," said Julia C. Owens, Ph.D., President and Chief Executive Officer of Millendo. "There are numerous etiologies of CS and, in patients for whom surgical intervention is not successful, current treatment options are limited. We are hopeful that the adrenal-selective effects of ATR-101 will provide a new treatment option for patients with endogenous CS, regardless of the underlying causes."

The Phase 2 clinical trial is a two-part multicenter study consisting of a 6-week open-label intra-subject dose-escalation period and a 4-week double-blind randomized withdrawal period to assess the efficacy and safety of orally-administered ATR-101 in patients with endogenous Cushing's syndrome. The primary efficacy endpoint will assess the impact of ATR-101 on urinary free cortisol (UFC) concentration, and secondary endpoints will evaluate the impact of ATR-101 on adrenal steroids/steroid intermediates and adrenocorticotrophic hormone (ACTH). The multicenter, international study will be conducted at sites in the U.S. and U.K. For additional information on this clinical trial, please visit [clinicaltrials.gov](#), identifier number NCT03053271.

About Endogenous Cushing's Syndrome

Endogenous Cushing's syndrome (CS) is a rare endocrine disorder characterized by increased cortisol production from the adrenal glands, which is the result of a pituitary, adrenal or ectopic tumor. The chronic cortisol excess in Cushing's syndrome can cause a multitude of issues for the patient such as weight gain, fatigue, hypertension, diabetes, bone loss, and neurologic symptoms. With chronic exposure to higher than normal levels of cortisol, patients may also exhibit cognitive impairment and mood disorders. In men, symptoms can also include decreased fertility and erectile dysfunction, while women may demonstrate menstrual irregularities and facial hair growth.

Endogenous CS affects approximately 20,000 people in the United States. It most commonly affects people who are 20 to 50 years of age, and women are three times more likely than men to have CS. Untreated CS can be life-threatening, and many cases can be cured through surgery, but for those cases that are refractory to surgery, medical management can be challenging. New broad-acting agents are needed, as currently approved treatments are only indicated for subsets of CS patients and are limited by side effects.

About ATR-101

ATR-101, an adrenal-selective small molecule inhibitor of ACAT1, reduces adrenal steroid production, and, at high doses, induces apoptosis of cells derived from the adrenal cortex. ATR-101 is currently in clinical development for the treatment of congenital adrenal hyperplasia (CAH), endogenous Cushing's syndrome (CS), and adrenocortical carcinoma (ACC).

About Millendo Therapeutics, Inc.

Millendo Therapeutics is focused on developing novel treatments for endocrine diseases caused by hormone dysregulation. Our mission 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. We are advancing two product candidates in five indications: MLE4901, designed to address Polycystic Ovary Syndrome (PCOS) and Vasomotor Symptoms (VMS), and ATR-101 for the treatment of Classic Congenital Adrenal Hyperplasia (CAH), Endogenous Cushing's Syndrome (CS), and Adrenocortical Carcinoma (ACC). www.millendo.com

###

Media Contact:

Casey R. Doucette, Ph.D.
MacDougall Biomedical Communications
+1 781 235 3060
cdoucette@macbiocom.com

Investor Contact:

Stephanie Ascher
Stern Investor Relations, Inc.
+1 212 362 1200
stephanie@sternir.com