FOR IMMEDIATE RELEASE

Millendo Therapeutics Announces Initiation of Phase 2 Clinical Trial of ATR-101 in Patients with Congenital Adrenal Hyperplasia

ANN ARBOR, Mich., June 8, 2016 – Millendo Therapeutics, Inc., a company developing novel therapies 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 classic congenital adrenal hyperplasia (CAH), a rare inherited endocrine disorder characterized by overgrowth of the adrenal glands. ATR-101, an adrenal-selective small molecule inhibitor of ACAT1, is also being studied in an ongoing Phase 1 clinical trial in patients with adrenocortical carcinoma (ACC).

“The initiation of this Phase 2 clinical trial of ATR-101 in classic CAH marks an important milestone in Millendo’s development and is the first of several planned trial initiations for this year as we build a portfolio of novel treatment options for endocrine diseases,” said Julia C. Owens, Ph.D., President and Chief Executive Officer of Millendo. “CAH is a serious condition with significant unmet need, where we believe we can provide an important new treatment option.”

“We look forward to exploring the potential of ATR-101 in classic CAH patients in this Phase 2 clinical trial,” said Richard J. Auchus, M.D., Ph.D., Professor of Internal Medicine and Pharmacology at the University of Michigan. “A great need exists for alternative treatment options, as the current standard of care for CAH can result in serious long-term side effects, including bone loss, growth impairment and Cushing’s syndrome. Based on the mechanism of action for this compound, we are optimistic that addition of ATR-101 to physiologic doses of corticosteroids can provide improved outcomes for these patients.”

The Phase 2 clinical trial is a multicenter, single-blind, multiple dose study that will assess the efficacy and safety of orally-administered ATR-101, in addition to corticosteroids, in patients with classic congenital adrenal hyperplasia resulting from 21-hydroxylase deficiency. The study is designed to determine an effective dose or range of doses with the primary efficacy endpoint assessing the impact of ATR-101 on adrenal steroid/steroid intermediate production as measured by serum 17-hydroxyprogesterone concentration. For additional information on this clinical trial, please visit http://clinicaltrials.gov.

About Congenital Adrenal Hyperplasia (CAH)
Congenital adrenal hyperplasia is a rare inherited endocrine disorder caused by a steroidogenic enzyme deficiency that is characterized by overgrowth of the adrenal glands, adrenal insufficiency and androgen excess. The most frequent form of CAH, responsible for 95% of cases, is a deficiency in the enzyme 21-hydroxylase, which is required for the production of cortisol in the adrenal glands. CAH can lead to severe virilization in females, testicular tumors in men, and infertility.

The incidence of classic CAH ranges from 1:10,000 to 1:20,000 births, and the current standard of care is treatment with corticosteroids, which are used to correct the endogenous cortisol deficiency and reduce androgen excess. However, high doses of corticosteroids are required to suppress androgens which can result in serious long-term side effects such as bone loss, growth impairment, and Cushing’s syndrome.

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 adrenocortical carcinoma (ACC) and congenital adrenal hyperplasia (CAH), with planned development in endogenous Cushing’s syndrome (CS).

About Millendo Therapeutics, Inc.
Millendo Therapeutics is focused on developing a portfolio of disease-modifying treatments for endocrine diseases caused by hormone dysregulation. Our product candidates seek to improve the quality of life for patients with orphan and specialty diseases with limited or no approved treatment options. Our clinical programs are designed to address:

- Polycystic Ovary Syndrome (PCOS) – the most common endocrine disease in women
- Congenital Adrenal Hyperplasia (CAH) – a recessive genetic defect of cortisol synthesis
- Endogenous Cushing's Syndrome (CS) – a condition resulting from chronic cortisol excess
- Adrenocortical Carcinoma (ACC) – a rare endocrine malignancy of the adrenal cortex

Our experienced team is committed to bringing these first-in-class therapies to market.
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