Press Release

AMO Pharma Announces First Patient Enrolled in REACH-CDM X 12-month Open-label Study

Sept 30, 2021

LONDON, Sept. 30, 2021 /PRNewswire/ -- AMO Pharma Limited ("AMO Pharma"), a privately held biopharmaceutical company focusing on rare, childhood-onset neurogenetic disorders with limited or no treatment options, today announced that the first patient has completed participation in the REACH-CDM pivotal clinical trial and has been enrolled in the REACH-CDM X study, a 52-week open-label study designed to evaluate the long-term safety and efficacy of AMO-02 (tideglusib), AMO Pharma's investigational therapy for the treatment of congenital myotonic dystrophy (DM1).

"The enrollment of the first patient in the REACH-CDM X open-label extension study for AMO-02 highlights the progress we are making with the REACH-CDM pivotal trial," said Joseph P. Horrigan, MD, AMO Pharma's chief medical officer. "Following participation in the REACH-CDM pivotal trial, patients are eligible to transition into the open-label trial where they will be treated with study drug for 12 months. This study will provide important additional insights that will help us better assess the long-term safety and efficacy profile of AMO-02."

The REACH-CDM X open-label study will include assessments of the long-term safety of treatment with AMO-02 as well as a range of efficacy measures. The REACH-CDM pivotal trial is a double-blind, placebo-controlled, randomized study in children and adolescents with congenital-onset myotonic dystrophy intended to support a future submission for marketing authorization in congenital myotonic dystrophy. The trial is currently enrolling a total of 56 patients at sites in the U.S., Canada, and New Zealand, with additional sites in Australia and other countries to be added pending local approvals. Patients will be assessed on a range of measures of CNS features and muscle function associated with CDM1 using a primary outcome measure agreed to by the FDA.

"We are very pleased to report this important milestone in our clinical program for DM1, which brings patients who have completed participation in our pivotal trial the opportunity to be treated with AMO-02 for 12 months," said Dr. Michael Snape, chief science officer at AMO Pharma. "We are grateful to our outstanding team of investigators and to all the patients and families who have expressed interest in participating in this trial and we are working tirelessly to advance this program to the final stages of clinical development."

Additional information about the REACH-CDM trial is available at https://www.reachcdm.com/.

About AMO-02
AMO-02 (tideglusib) is in development for the treatment of congenital myotonic dystrophy and has potential for use in additional CNS, neuromuscular and other orphan indications. AM0-02 is a clinical stage investigational medicine for the treatment of the severe form of congenital myotonic dystrophy known as DM1 or Steinert disease. AMO-02 has a dual mechanism disrupting the pathogenic RNA repeat in CDM1 and inhibiting excess levels of the kinase GSK3β.

About AMO Pharma
AMO Pharma is a biopharmaceutical company working to identify and advance promising therapies for the treatment of serious and debilitating diseases in patient populations with significant areas of unmet need, including rare and severe childhood onset neurogenetic disorders with limited or no treatment options. In addition to developing AMO-02 for congenital myotonic dystrophy, the company is also progressing AMO-01 as a clinical stage treatment for Phelan-McDermid syndrome and AMO-04 as a clinic-ready potential medicine for Rett syndrome and related disorders. AMO-02, AMO-01 and AMO-04 are investigational medicines that have not yet been approved for the treatment of patients anywhere in the world. For more information, please visit the AMO Pharma website at http://www.amo-pharma.com/.

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