

AMO Pharma Announces Initiation of Pivotal REACH-CDM Clinical Trial for AMO-02 in Treatment of Congenital Myotonic Dystrophy

Following collaborative review of amended protocol with FDA to address clinical study operations associated with COVID-19 pandemic, trial to be conducted at multiple sites globally

LONDON, December 22, 2020 – 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 the initiation of REACH-CDM, a pivotal clinical study to assess the efficacy and safety of AMO-02, the company's dual action investigational mRNA modulating/GSK3 β kinase inhibitor, in the treatment of congenital myotonic dystrophy (CDM1).

Initiation of the study follows the successful completion of discussions with the U.S. Food and Drug Administration (FDA) regarding amendments to the clinical trial protocol to address potential issues associated with execution of the study presented by the COVID-19 pandemic and approval by Health Canada of the revised study protocol. These discussions occurred in addition to a recent end of phase 2 meeting conducted with FDA, and the FDA decision to grant Pediatric Rare Disease designation to AMO-02 for the treatment of CDM1.

"COVID-19 has caused disruption in clinical research for almost every drug developer around the world. Based on our experience with clinical studies in pediatric neurological disorders, we were able to outline a path forward that both optimizes safety for participants in the REACH-CDM study and positions us to provide regulators with a clear and unambiguous assessment of outcomes," said Dr. Joseph Horrigan, AMO Pharma chief medical officer. "We are grateful that FDA has agreed to our approach of using telehealth as a strategy to manage the potential impact of COVID-19 on the progression of this important study."

Dr. Aravindhan Veerapandiyan, of Arkansas Children's Hospital, said "We are extremely excited to start enrolling patients for REACH-CDM, a pivotal therapeutic trial in the space of congenital myotonic dystrophy. The unique aspects of this research trial that interest me are utilization of telehealth as well as the primary outcome measure used to assess the treatment response. I am hopeful that this investigational product can be a life changer for patients living with this ultra-rare severe muscular dystrophy and their families."

The REACH-CDM study was designed based on the results of positive Phase 2 data that were recently published in the peer-reviewed journal *Pediatric Neurology*. In prior research, AMO-02 has been shown to access brain, muscle and other tissues and reduce the effect of DMPK expansion repeat in mRNA that is the pathological basis for congenital myotonic dystrophy. REACH-CDM 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 will seek to enroll a total of 56 patients initially at sites in the U.S. and Canada, with additional sites in Australia, New Zealand and other countries to be added pending local approvals. AMO Pharma will issue further updates from Q1 of next year. 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.

“Congenital myotonic dystrophy is a devastating disease that has a profound impact on the health and quality of life of patients and families. We are very encouraged by the levels of interest in the REACH-CDM trial from investigators and researchers and from families who are so anxious for a treatment,” said Ibs Mahmood, AMO Pharma CEO, adding, “We are dedicated to advancing the development program for AMO-02 as rapidly as possible and are grateful to the regulators, investigators and leaders in patient advocacy for their support.”

Additional information about the REACH-CDM trial is available at www.clinicaltrials.gov.

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. AMO-02 is a clinical stage investigational medicine for the treatment of the severe form of congenital myotonic dystrophy known as DM1 or Steinert disease. In cellular and animal models of DM1 as well as in muscle biopsies from patients, activity of glycogen synthase kinase 3 beta (GSK3 β) has been shown to increase. AMO-02 is an inhibitor that has been shown to normalise levels of GSK3 β in transgenic models and in ex vivo tissue samples in patients with DM1 and to reduce levels of the mRNA that is pathogenic for DM1.

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/>.

Contacts

Media:
Bill Berry
Berry & Company Public Relations
212.253.8881
bberry@berrypr.com

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