Press Release

AMO Pharma Announces Completion of Treatment of Last Patient in Pivotal REACH-CDM Clinical Trial in Myotonic Dystrophy

Company on track to present first data from REACH-CDM trial in July 2023.

April 25, 2023

LONDON, April 25, 2023 /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 completion of treatment for the final patient enrolled in the company's REACH-CDM clinical study of the investigational therapy AMO-02. 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.

“Completion of the treatment phase of the REACH-CDM study represents an important milestone for people around the world affected by DM1 and for our team of researchers and investigators who have worked so tirelessly to advance this program including addressing the challenges presented by the COVID pandemic,” said Dr. Ibs Mahmood, Chief Executive Officer at AMO Pharma. “We are very pleased that we successfully achieved our target enrollment and look forward to presenting the first data from this study in July 2023.”

The REACH-CDM study met its planned enrollment of a total of 56 patients at sites in the U.S., Canada, Australia and New Zealand. AMO Pharma continued to advance the study during the COVID pandemic with strategies including use of telehealth options to support the highest levels of patient safety without compromising the trial results. Following completion of treatment in the study, patients have the option to continue treatment through the ongoing REACH-CDM X extension study.

“The fact that we achieved our target enrollment for the study and have now completed treatment for the final enrolled patients brings new levels of hope to families around the world affected by this devastating genetic condition,” said Dr. Nicholas Johnson, a Professor at the Department of Neurology, Virginia Commonwealth University School of Medicine, Principal Investigator in the REACH-CDM study. “We look forward to continuing to support this landmark research effort and to presenting data from the study in the months ahead.”

“We are very grateful to all the patients, families, investigators and patient advocates and advocacy groups who have supported this research, often through very challenging conditions presented by the COVID pandemic,” said Dr. Mahmood. “Completion of treatment for the final patient enrolled in the study brings us new levels of optimism and enthusiasm as we plan for the final stages of this development program with the potential to deliver a first approved treatment for DM1.”

AMO Pharma will provide an update on the REACH-CDM trial during the “Meet the Drug Developers” webinar sponsored by the Myotonic Dystrophy Foundation on July 7, 2023. For information and to register visit https://register.gotowebinar.com/register/4243790956756657494.

About AMO-02

AMO-02 (tideglusib) is in development for the treatment of congenital myotonic dystrophy and has potential for use in adult-onset myotonic dystrophy, additional central nervous system (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 CDM1 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. Advice provided to AMO Pharma by UK MHRA is under the condition that any scientific advice given is not legally binding with regards to any future application for the product concerned, neither on the part of MHRA/Commission on Human Medicines (CHM) nor on the Company. Furthermore, advice cannot be taken as indicative of any future agreed position.

For more information, please visit the AMO Pharma website at http://www.amo-pharma.com/.

Media contact:
Caitlin O'Reilly
Berry & Company Public Relations
coreilly@berrypr.com
212-253-8881

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