Press Release

AMO Pharma Announces MHRA Confirmation of CDM1-RS as Approvable Primary Outcome Measure in Myotonic Dystrophy

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Opinion follows previous development and agreement with the FDA on CDM1-RS as a suitable primary endpoint in AMO's Phase 3 clinical trial for congenital myotonic dystrophy; measure has ability to assess treatment benefit across multiple symptom areas.

LONDON, Feb. 27, 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 that the UK Medicines and Healthcare products Regulatory Agency (MHRA) has confirmed that the Congenital DM1 Rating Scale (CDM1-RS) can be considered an approvable primary outcome measure in clinical trials to develop a treatment for congenital myotonic dystrophy type 1 (CDM1).

The CDM1-RS is a derivative of the Myotonic Dystrophy Health Index (MDHI) and childhood version (ccMDHI) developed by Professor Chad Heatwole and Professor Nicholas Johnson and owned and licensed by the University of Rochester. Following advice from the U.S. Food and Drug Administration (FDA), the scale was incorporated by AMO Pharma into the REACH-CDM pivotal study in CDM1. The CDM1-RS is a fit-for-purpose clinician administered scale able to quantify 11 observable characteristics of CDM1 and rate each on a four-point Likert scale. The MHRA reviewed data from AMO Pharma on how the scale was developed using information from natural history studies, consultation with therapeutic area experts and feedback from patient advocates. AMO Pharma showed an evaluation of the scale's use in clinical studies, proposing it has potential to effectively assess the CDM1 phenotype in a low-burden manner, is change sensitive and can therefore detect treatment effects, and may provide useful outcome measures for future clinical trials. The MHRA concluded the measure could be considered an approvable primary outcome measure in the event of a positive result in the REACH-CDM study of AMO-02 in CDM1.

The MHRA provided scientific advice to AMO Pharma for the program, which was granted Innovation Passport and Innovative Licensing and Access Pathway (ILAP) status in the UK in July 2021. The MHRA launched the ILAP program in January 2021 to support innovative approaches to the safe, timely and efficient development of promising medicines to

improve patient access in the UK. The Innovation Passport, a new medicine designation, is the first step in the ILAP process, triggering the MHRA and its partner agencies, including the National Institute for Health and Care Excellence (NICE), the Scottish Medicines Consortium (SMC), the All Wales Therapeutics and Toxicology Centre and National Health Service (NHS) England, to create a target development profile that outlines a unique product-specific roadmap for regulatory and development milestones with the goal of early patient access in the UK. The Target Development Profile (TDP) under ILAP was agreed to in 2022.

"CDM1-RS was developed in an effort to support a more comprehensive assessment of treatment benefit in DM1 clinical research, including congenital DM1," said Dr Ibraheem Mahmood, Chief Executive Officer at AMO Pharma. "This approach can play a central key role in advancing efforts to develop new treatments in the years ahead. We are grateful to the MHRA for their helpful and clear advice."

Peter Ashley, Chairperson of Cure DM, a nonprofit advocacy organization representing individuals impacted by congenital myotonic dystrophy, said, "DM affects the whole body in complex ways and children with congenital DM1 are affected most severely. For them, measures that are often used in clinical trials have little meaning or simply cannot be done. Their daily difficulties are the inability to think clearly, communicate their needs, or eat without choking. CDM1-RS provides measures that can show whether a new treatment will have an impact where it really matters to people living with DM1 and their caregivers."

In December 2022 AMO Pharma announced completion of patient enrollment in the company's REACH-CDM study of the investigational therapy AMO-02 (tideglusib) for the treatment of congenital myotonic dystrophy. 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. CDM1-RS is the primary outcome measure in the REACH-CDM clinical trial. The company expects topline data from the study in mid-2023.

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/.

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