Press Release

AMO Pharma Completes Meeting with U.S. FDA and Outlines Plans to Advance Clinical Development of AMO-02 (tideglusib) in Treatment of Myotonic Dystrophy

Company will conduct Phase 3 trial of AMO-02 (tideglusib) in adults to support a future submission for marketing authorization in Type 1 myotonic dystrophy in both adult and pediatric indications.

May 2, 2024

LONDON, May 2, 2024 – AMO Pharma Limited ("AMO Pharma"), a privately held clinical-stage specialty biopharmaceutical company focusing on rare genetic disorders with limited or no treatment options, today provided an update on plans to advance the clinical development program for AMO-02 (tideglusib), the company's investigational therapy in development for the treatment of Type 1 myotonic dystrophy (DM1), following a recent meeting with the U.S. Food and Drug Administration (FDA). Based on the input from the meeting, AMO Pharma will conduct a Phase 3 clinical trial to assess the efficacy and safety of AMO-02 in adult patients with adult-onset DM1 to support a future submission for marketing authorization for the treatment of DM1 in children, adolescents and adults.

Results from AMO Pharma's previous REACH-CDM Phase 2/3 clinical study (the "REACH-CDM Study") involving children and adolescents with congenital-onset DM1 showed that *AMO-02 was generally safe and well tolerated during the study, with no reported severe adverse events (SAEs) related to treatment. The primary and secondary outcome measures based on clinician administered subjective ratings showed an unexpected placebo effect that may have masked some beneficial effects of treatment. Further post hoc analysis as reviewed with the FDA indicates that there is sufficient evidence to support the continued development of tideglusib for the treatment of DM1 with agreement that AMO Pharma will now advance a Phase 3 study in adults with the adult-onset version of DM1.*

During the meeting, FDA reviewed data from the REACH-CDM Study to assess the efficacy and safety of treatment with AMO-02 in children and adolescents with congenital DM1. The FDA also gave feedback on the design of the new Phase 3 clinical trial for adult patients with adult onset DM1. In this study in adult onset DM1, outcome measures will be similar to those that showed benefit in the REACH-CDM Study. Following discussion with the FDA, AMO Pharma will plan a future submission for approval in children, adolescents and adults with myotonic dystrophy based on a package of safety and efficacy data from

the new Phase 3 trial in adults combined with efficacy, pharmacokinetic and safety data from AMO Pharma's REACH-CDM Study.

"We are pleased with the outcome of the productive discussions we had with the FDA regarding next steps for AMO-02 and continue to feel strongly that this innovative therapy can represent a significant advance in the treatment of patients with DM1, whether they are children, adolescents or adults," said Alan Rubino, executive chair at AMO Pharma. "We are now focused on progressing a new clinical study in adults with adult-onset DM1. We are very grateful to the FDA and to the patients, families, clinicians and investors who have supported our research thus far and we look forward to working with the community to advance this program as rapidly as possible. We will make an announcement regarding the start date and site locations for the adult onset DM1 study shortly."

"The results of the REACH-CDM Study showed measures of efficacy benefit across multiple symptom areas, and we are very excited that this clinical development program will continue to move forward," said Hanns Lochmuller, investigator in the REACH-CDM Study at Children's Hospital of Eastern Ontario, Canada. "We remain very hopeful that this investigational therapy can have a transformative impact on the health of patients living with this ultra-rare, severe muscular dystrophy in the years ahead."

About AMO-02

AMO-02 (tideglusib) is an investigational therapy in development for the treatment of DM1 with a dual mechanism disrupting the pathogenic RNA repeat in DM1and inhibiting excess levels of the kinase GSK3β.

The REACH-CDM Study was a double-blind, placebo-controlled, randomized study in children and adolescents with congenital DM1 intended to support a future submission for marketing authorization in congenital DM1. The study included 56 participants at leading specialist sites in the US, Canada, New Zealand and Australia.

Data associated with functional and objective measures were collected during the study, including motor and muscle assessments (10 meter walk/run, myometry, lip strength and lean muscle mass as assessed by DXA scan), cognitive performance (Peabody Picture Vocabulary Test, NIH Toolbox Picture Sequence Memory Test, NIH Toolbox Dimensional Change Card Sort Test), adaptive (real world) function (Vineland Adaptive Behavior Communication Scale, Vineland Adaptive Behavior Daily Living Scale, Vineland Adaptive Behavior Socialization Scale), bone density (DXA scan) and creatine kinase measurements. In post-hoc analyses more participants showed a positive response following AMO-02 treatment than placebo on 10 of the 12 of these measures. Treatment with AMO-02 was associated with clinically significant improvements in walking and cognition and significant effects on objective biomarkers of muscle and neuronal integrity. AMO-02 also showed significant improvement compared to placebo on a Multi-Domain Responder Index integrating clinically significant responses on measures of ambulation, muscle strength, cognitive performance, activities of daily living and an objective biomarker. Primary analyses of subjective rating assessments showed a placebo effect. Patients from this study and treatment naïve patients with congenital or juvenile onset DM1 are currently being supported in the REACH CDM-X open label extension safety study.

About AMO Pharma
AMO Pharma is a clinical-stage specialty 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 DM1, 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 regulators is under the condition that any scientific advice given is not legally binding with regards to any future application for the product concerned. 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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