Press Release

AMO Pharma Announces Affirming Data from REACH-CDM Clinical Trial for AMO-02 in Treatment of Myotonic Dystrophy

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Results to be reviewed during 2023 Myotonic Dystrophy Annual Conference show statistically and clinically significant efficacy benefits in multiple areas including cognitive performance, reduction in biomarkers and improvements in walk/run measurements. AMO-02 was safe and well tolerated during the study with no SAEs related to treatment. Primary outcome measure based on clinician administered subjective rating showed an unexpected placebo effect that masked beneficial effect of treatment.

Company planning meetings with regulators to outline path forward in development of AMO-02 including plans for study in patients with adult-onset myotonic dystrophy.

LONDON, Sept. 6, 2023 /PRNewswire/ -- AMO Pharma Limited ("AMO Pharma"), a privately held clinical-stage specialty biopharmaceutical company focusing on rare childhood-onset neurogenetic disorders with limited or no treatment options, today announced results from the company's REACH-CDM clinical study of the investigational therapy AMO-02 in the treatment of children and adolescents with congenital myotonic dystrophy. Results will be reviewed on Saturday, September 9 at 3:30 PM ET in a briefing event during the 2023 Annual Meeting of the Myotonic Dystrophy Foundation being held at the Renaissance Washington DC Downtown Hotel in Washington, DC September 7-9, 2023.

Topline results from the study based on a clinician administered rating instrument intended to canvas the entire clinical phenotype of congenital DM1 showed a positive benefit in both the treatment and placebo groups, which may be attributed to difficulties in patient monitoring and compliance with reporting protocols caused by the COVID pandemic. While the study did not meet the primary endpoint of a statistically significant benefit over placebo based on the FDA-authorized physician-completed rating scale, clinically and statistically significant benefit was achieved during analysis of a range of functional and objective assessments in the treatment group compared to placebo. Treatment with AMO-02 was associated with clinically significant improvements in cognitive performance (Peabody Picture Vocabulary Test, $p<0.05$), reduction in a widely used biomarker of skeletal and cardiac muscle integrity (creatine phosphokinase, $p<0.05$) and improvement in the 10m walk/run test ($p=0.054$). A composite statistical analysis of

outcomes assessing motor skills, muscle strength, cognitive ability, daily living skills and biomarker data showed a statistically significant benefit of treatment with AMO-02 compared to placebo ($p<0.05$). The benefits seen with AMO-02 were related to pharmacokinetic parameters showing that increased plasma levels of AMO-02 resulted in greater clinical improvement.

"We are very encouraged by the consistent benefit shown across multiple clinically confirmed measures of efficacy," said Dr. Joe Horrigan, Chief Medical Officer at AMO Pharma. "These data reflect a broad profile of benefit in cognitive, motor, muscle, real world adaptive behavior and biochemical measures associated with treatment with no reported treatment-related serious adverse events."

Patients enrolled in the REACH-CDM trial were invited to continue treatment in an open-label extension study (OES) and 98% of patients opted to continue treatment in that study. Patients were again given the option of continuing treatment at the conclusion of the 1-year OES study and 85% of patients opted to continue treatment.

"We are immensely grateful to all the families who took part in this study, patient advocates in the myotonic dystrophy community, investigators who worked with us to face the challenges presented by the COVID pandemic, and our investors for supporting this work," said Dr. Ibs Mahmood, Chief Executive Officer at AMO Pharma. "These results provide strong further validation of the potential benefits of treatment with AMO-02 in multiple key areas that represent the most severe symptoms and disabilities associated with DM1. We are now working to discuss next steps with regulators in order to advance this programme."

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.

The REACH-CDM pivotal trial was 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 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), and bone density (DXA scan) and creatine kinase measurements. More participants showed a positive response following AMO-02 treatment than placebo on 10 of the 12 quantifiable measures.

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 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 regulators 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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