Press Release

AMO Pharma Reports Long-Term Safety Data from REACHCDM-X Study of AMO-02 in Treatment of Congenital Myotonic Dystrophy Type 1

-Four years of study data highlight continued favorable safety profile for AMO-02
-FDA meeting planned in Q4 2025 to discuss potential route to NDA submission for congenital myotonic dystrophy

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LONDON, Sept. 15, 2025 /PRNewswire/ -- 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 announced a preliminary analysis of safety outcomes from the company's ongoing REACHCDM-X open-label extension (OLE) study of AMO-02 for the treatment of congenital and childhood-onset myotonic dystrophy type 1 (DM1).

"We are pleased to share new findings that reflect the experience of patients treated with AMO-02 for almost four full years in our REACHCDM-X open-label extension study. These results include new insights indicating that AMO-02 continues to have an advantageous safety profile, and we were especially encouraged by the low rate of hospitalizations observed during the study," said Mike Snape, Chief Executive Officer at AMO Pharma. "We look forward to meeting with the FDA later this year to review the most recent data and our plans to advance AMO-02 for the treatment of congenital myotonic dystrophy as quickly as possible."

Key findings from the REACHCDM-X OLE study of AMO-02 include:

- AMO-02 has been generally safe and well-tolerated over four years of study.
- As of August 2025, 45 participants remain on treatment, including 20 who have received AMO-02 for more than three years. A total of 14 participants discontinued treatment, with only one withdrawal due to an adverse event (elevated liver enzymes).
- Most adverse events were mild or moderate, with respiratory infections and gastrointestinal events being the most common. These are consistent with the known natural course of congenital and childhood-onset DM1.

- Across more than 151 patient-years of treatment exposure, participants treated with AMO-02 experienced a hospitalization rate of only 0.14 events per patient per year.
- No deaths or cardiovascular events have been reported.
- In a 10-meter walk/run test, a secondary endpoint in the trial, participants aged 10 years and older showed little or no decline over one year of treatment.

AMO Pharma has submitted these additional data for AMO-02 to the U.S. Food and Drug Administration (FDA) and plans to submit to Health Canada and the UK Medicines and Healthcare products Regulatory Agency (MHRA). AMO Pharma is also scheduled to meet with the FDA in Q4 2025 to discuss the development plan for AMO-02 in the treatment of congenital myotonic dystrophy including assessments on the use of real-world evidence to support a regulatory submission. AMO Pharma has also submitted a protocol for a Phase 3 study in adult-onset myotonic dystrophy to the FDA and will share updates on this review, as well as on the outcomes of the upcoming FDA meeting and ongoing regulatory discussions. AMO Pharma is also planning meetings with UK MHRA and Health Canada.

"We are very encouraged by these data, which significantly expand our understanding of the safety and potential clinical benefit of AMO-02 in congenital myotonic dystrophy," said Dr. Snape. "For families affected by congenital and childhood-onset DM1, this disorder is often associated with life-threatening complications. AMO Pharma is encouraged by these data, and we look forward to discussions with regulatory health agencies about the potential to evaluate real-world evidence outcomes that are highly relevant for this community, and may shape the nature of future investigations."

The REACHCDM-X study, which began in August 2021 as an open-label extension of the double-blind REACHCDM trial, is the largest and longest-running interventional study ever conducted in congenital and childhood-onset myotonic dystrophy. The study has also expanded to include participants with childhood-onset DM1 up to 45 years of age and offers an optional extended access component, allowing patients to continue on long-term therapy.

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-02 as a clinical stage treatment for Arrhythmogenic Right Ventricular Cardiomyopathy (ARVC). AMO-01 is being investigated for treatment of 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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