



Press Release

AMO Pharma Announces Update on Scientific Advice for Registrational Clinical Study of AMO-02 in Congenital Myotonic Dystrophy Type 1 Following Meetings with the U.S. Food and Drug Administration, the U.K. Medicines and Healthcare products Regulatory Agency and Health Canada

News provided by
[AMO Pharma Limited](#)
Jul 06, 2026, 17:30 ET

Based on regulatory agency feedback, the Company plans a registrational study that will evaluate hospitalization as the primary outcome measure, supported by multiple functional assessments as secondary outcome measures.

LONDON, July 6, 2026 /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 agreement with regulatory agencies in the U.S., U.K. and Canada on design for a registrational clinical study intended to assess the safety and efficacy of the Company's investigational therapy AMO-02 (oral tideglusib) in the treatment of congenital myotonic dystrophy type 1 (cDM1).

The design requirements and primary outcome choice for this registrational study are based on advice provided to AMO Pharma by the U.S. Food and Drug Administration (FDA), the U.K. Medicines and Healthcare products Regulatory Agency (MHRA) and Health Canada following meetings over the last six months. AMO-02 is an investigational compound that has not been approved by any regulatory authority. To date, the safety and efficacy of AMO-02 have not been established.

Based on this regulatory advice, the registrational study for AMO-02 is expected to use hospitalization as the primary efficacy endpoint. Hospitalization represents a significant burden for people living with cDM1 and their caregivers, as cDM1 is a potentially life-threatening disorder associated with multiple medical co-morbidities. AMO Pharma also plans to engage the cDM1 community through a survey to better understand the impact of symptoms and hospitalizations associated with this disorder. The clinical study will also include a range of functional assessments as secondary outcome measures to help

characterize disease progression and capture key features of cDM1, which can vary widely in its clinical presentation.

"We appreciate the thoughtful engagement of FDA, MHRA and Health Canada throughout this important process and are pleased to have received feedback that informs the design of the planned registrational study for AMO-02," said Dr. Mike Snape, chief executive officer at AMO Pharma. "With insights from these regulatory agencies, we are now well positioned to plan and execute a registrational study as quickly as possible. For individuals and families affected by cDM1, the need for new treatment options remains critical, and we look forward to updating on our progress in advancing this program in the months ahead."

Congenital DM1 is a rare, inherited neuromuscular disorder that can cause serious and lifelong challenges affecting muscle function, learning difficulties, development, cardiac problems and overall health. Many affected individuals experience serious complications requiring hospitalization and ongoing multidisciplinary care. There are currently limited treatment options available for individuals living with the condition.

"Families affected by cDM1 carry an extraordinary burden, often managing serious health challenges from the earliest days of life," said Lisa Harvey-Duren, a leading patient advocate in DM1 and a consultant for AMO Pharma. "Alignment on a trial design that reflects clinically meaningful outcomes is an important methodological step in evaluating potential therapies for cDM1. The selection of patient-relevant outcome measures is an important consideration in clinical research design for congenital myotonic dystrophy."

The scientific advice received by AMO Pharma from regulators is consistent with the language regarding cDM1 adopted in the FY27 Agriculture, Rural Development, Food and Drug Administration, and Related Agencies Appropriations bill.

"Having participated in the REACHCDM-X study of AMO-02 over the last four years, I welcome this advice from regulators in Canada, the U.S. and the U.K., as the field looks to plan future interventional studies in cDM1, which is a serious and underserved condition," said Professor Hanns Lochmuller, principal investigator in the REACH-CDM study at the Children's Hospital of Eastern Ontario, in Ottawa, Canada.

The Company expects to provide an update on the planned initiation of the study during the third quarter of 2026.

About AMO Pharma

AMO Pharma is a clinical-stage specialty biopharmaceutical company working to identify and advance investigational 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). 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. This press release contains forward-looking statements regarding anticipated trial initiation and development plans. Such statements are subject to risks and

uncertainties, including regulatory, clinical, and operational factors, that could cause actual results to differ materially.

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

Media contact:

Holly Stevens

CG Life

hstevens@cglife.com

SOURCE AMO Pharma Limited