Press Release

AMO Pharma Announces Expansion of Pivotal REACH-CDM Study in Congenital Myotonic Dystrophy

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- Additional research centers now enrolling patients in Australia and New Zealand
- Company has achieved 50 percent of target enrollment in global Phase 3 trial

LONDON, December 22, 2021 /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 the completion of activation of two additional clinical trial sites in Australia and New Zealand for its pivotal REACH-CDM study evaluating the efficacy and safety of the investigational therapy AMO-02 (tideglusib) in the treatment of congenital myotonic dystrophy (CDM1). The company also announced that the trial has recently passed 50 percent enrollment.

“CDM1 is the most severe and earliest occurring form of myotonic dystrophy, with patients often experiencing cognitive and critical thinking challenges, speech impairment, muscle weakness and autism-like symptoms beginning shortly after birth,” said Dr. Joseph Horrigan, Chief Medical Officer at AMO Pharma. “As a physician focused on studying pediatric neurogenetic diseases, it is especially exciting to initiate this landmark clinical trial for CDM1 in patients who reside in Australia and New Zealand, which represents a significant advance in research to develop an effective treatment for this underserved community.”

The REACH-CDM trial now has multiple sites enrolling patients in the U.S., Canada, New Zealand and Australia, with additional sites planned. The recently activated participating centers are:

- New Zealand Clinical Research (NZCR), Auckland, New Zealand
  Principal investigator: Dr. Gina O'Grady
- Sydney Children's Hospital Network, Randwick, New South Wales, Australia
  Principal investigator: Dr. Michelle Farrar

The REACH-CDM trial will enroll a total of 56 patients. Upon completion of the trial, patients will have the opportunity to transition to the REACH-CDM X study, a 52-week open-label study designed to evaluate the long-term safety and efficacy of AMO-02.

“Achievement of 50% enrollment is a key milestone towards completion of the REACH-CDM trial and our efforts to advance the development of AMO-02 as a treatment for congenital myotonic dystrophy,” said Dr. Michael Snape, Chief Scientific Officer at AMO Pharma. “We are very proud of our progress to date. With 12 global research centers now actively recruiting, we also very much appreciate the support from our outstanding team of investigators as well as the patients and caregivers for their continued commitment to participating in this study despite the challenging circumstances caused by the COVID-19 pandemic.”

To learn more about the REACH-CDM trial including a list of treatment centers, visit www.reachcdm.com.

About AMO-02
AMO-02 (tideglusib) is in development for the treatment of congenital myotonic dystrophy and has potential for use in additional 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. For more information, please visit the AMO Pharma website at http://www.amo-pharma.com/.

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