Press Release

AMO Pharma Announces Completion of Enrollment in REACH-CDM Study in Congenital Myotonic Dystrophy

Dec 6, 2022

Company also announces additional private equity investment

LONDON, Dec. 6, 2022 /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 completion of patient enrollment in the company's REACH-CDM study of the investigational therapy AMO-02 in treatment of congenital myotonic dystrophy. The REACH-CDM pivotal trial is 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.

"Completion of enrollment represents a significant milestone for both AMO Pharma and the patients and families affected by congenital myotonic dystrophy around the world," said Dr. Joseph Horrigan, Chief Medical Officer at AMO Pharma. "We are very grateful to the patients, caregivers and leaders from the CDM advocacy community who have helped build awareness of this study and to the outstanding team of investigators who are dedicated to advancing this historic research effort and bringing a new disease-modifying therapy to CDM patients."

The Company also announced additional investment in AMO Pharma in response to progress in the REACH-CDM study. Funding will support AMO Pharma activities following completion of the REACH-CDM study.

"We are building new levels of momentum in advancing our development program and are grateful for the continued levels of investor support and enthusiasm," said Ibs Mahmood, Chief Executive Officer at AMO Pharma. "We remain dedicated to continuing to work with the leaders from the research and advocacy communities in completing the REACH-CDM trial and advancing our other promising development programs in the months ahead."

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 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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