Press Release

AMO Pharma Awarded Innovation Passport for AMO-02 (Tideglusib) for Treatment of Congenital Myotonic Dystrophy Type 1

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Company's lead clinical candidate receives innovative medicine designation under new program designed to accelerate drug approval process and improve patient access in United Kingdom

LONDON, August 17, 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 that the Company has received innovative licensing and access pathway (ILAP) designation from the UK Medicines and Healthcare products Regulatory Agency (MHRA) for AMO-02 (tideglusib), an investigational therapy in development for the treatment of congenital myotonic dystrophy type 1 (CDM1).

The MHRA launched the ILAP program in January 2021 to support innovative approaches to the safe, timely and efficient development of promising medicines to improve patient access in the UK. The Innovative Passport, a new medicine designation, is the first step in the ILAP process, triggering the MHRA and its partner agencies, including the National Institute for Health and Care Excellence (NICE), the Scottish Medicines Consortium (SMC) and National Health Service (NHS) England, to create a target development profile that outlines a unique product-specific roadmap for regulatory and development milestones with the goal of early patient access in the UK. More information about the ILAP program can be found here: https://www.gov.uk/guidance/innovative-licensing-and-access-pathway.

This Innovation Passport designation is among the first ever granted to a company developing promising therapies for diseases of the central nervous system including developmental disorders such as CDM1, showing that the MHRA recognizes the urgent need for a treatment option for these patients," said Joseph Horrigan, MD, chief medical officer at AMO Pharma. "The MHRA's decision is an important step in our efforts to bring a potential treatment to CDM1 patients living in the UK as rapidly as possible and we look forward to continued collaboration with the agency and its partners as we advance our AMO-02 clinical development program."

CDM1 is a rare genetic disease that presents itself at birth and is characterized by cognitive and physical impairment. Symptoms can include autism spectrum symptoms including difficulty with thinking and problem solving, weakened muscles and speech, and hearing and vision difficulties. There are currently no approved therapies for CDM1. Patients are typically treated with therapies to address different symptoms of the disease. Many patients also receive support through special education and speech and physical therapy. AMO-02 accesses brain, muscle and other tissues and has been shown to reduce the DMPK expansion repeat mRNA that is the pathological basis for CDM1. In addition to Innovation Passport designation, AMO-02 has received Fast Track and Rare Pediatric Disease Designations by the U.S. Food and Drug Administration (FDA).

“Congenital myotonic dystrophy is a devastating disease that significantly affects the quality of life of patients, their families and caregivers. It is encouraging to see the MHRA grant this new designation to AMO-02, targeting the underlying cause of the disease and recognising the urgent need for new therapies for those currently living without any treatments,” said Peter Ashley of Cure DM UK Charity. “This is an important milestone for patients and families in the UK, giving hope that a new treatment might soon be available to our DM community around the world.”

AMO-02 is currently being assessed in an ongoing pivotal trial, called the REACH-CDM study, that is being conducted at 11 treatment centers globally and plans to enroll a total of 56 patients who will be assessed on a range of measures of CNS features and muscle function associated with CDM1. For more information on REACH-CDM, visit https://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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