MARCH 2021

AMO Update

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Moving Forward: The REACH-CDM Trial in Congenital Myotonic Dystrophy

In 2020, COVID-19 presented significant challenges for companies and healthcare providers involved in clinical research all around the world. Many research teams were impacted and have had to work closely with the FDA and other regulatory agencies to amend trial protocols to keep patients and clinicians safe while also collecting the necessary levels of data on safety and efficacy. In January 2020, AMO Pharma was very pleased to announce the initiation of the pivotal REACH-CDM clinical trial with AMO-02 in the treatment of congenital myotonic dystrophy. When the global COVID-19 pandemic began just a few weeks later, we immediately initiated a plan of action to identify the best strategy to keep this landmark research effort moving forward:

- Our team quickly recognized the impact that COVID-19 would have on the ability of patients and healthcare providers to participate in the REACH-CDM trial.
- We began rapidly to consider different approaches that could maintain the highest levels of safety and convenience for participants in the trial while also helping us collect the levels of data needed for a regulatory filing. In this article from Clinical Leader, AMO Pharma Chief Scientific Officer Dr. Mike Snape shares more about our efforts to keep patients and caregivers safe while conducting clinical trials during the COVID-19 pandemic.

"We are extremely excited to start enrolling patients for REACH-CDM. I am hopeful that this investigational product can be a life changer for patients living with this ultra-rare severe muscular dystrophy and their families."

Dr. Aravindhan Veerapandiyan
Principal Investigator,
Arkansas Children's Hospital

REACH CDM

- Throughout this effort, we worked in close collaboration with leaders from the U.S. Food and Drug Administration (FDA) and Health Canada to identify opportunities to amend the protocol for the REACH-CDM trial to effectively address the challenges associated with COVID-19. Working together, we were able to develop an amended protocol including fail safes such as telehealth designed to offer maximum safety and convenience for patients, caregivers and clinical trial sites participating in this trial.

"We were able to outline a path forward that both optimizes safety for participants in the REACH-CDM study and positions us to provide regulators with a clear and unambiguous assessment of outcomes."

Joseph Horrigan, MD
Chief Medical Officer

- In September 2020, AMO Pharma leadership presented an update on the company's research targeting use of CNS therapies in the treatment of myotonic dystrophy in an industry update during the virtual Myotonic Dystrophy Foundation Annual Conference. This meeting was an important opportunity for patients, caregivers and clinicians to learn firsthand about AMO Pharma's efforts to advance this important research program during the pandemic and meet with members of our team in a virtual setting.
- In November we announced that the FDA granted a Rare Pediatric Disease (RPD) designation to AMO Pharma for AMO-02 for the treatment of congenital myotonic dystrophy.
- In December 2020 we announced the initiation of the REACH-CDM trial based on the amended protocol developed in collaboration with the FDA and with approval to proceed from Health Canada.

continued on page two

Moving Forward: The REACH-CDM Trial in Congenital Myotonic Dystrophy

continued from page one

REACH-CDM is a double-blind placebo controlled randomized study intended to support a future submission for marketing authorization for the use of AMO-02 in the treatment of congenital myotonic dystrophy. The trial will seek to enroll a total of 56 patients initially at sites in the U.S. and Canada, with additional sites in Australia, New Zealand and other countries to be added pending local approvals. Patients will be assessed on a range of measures of CNS features and muscle function associated with CDM1, using a primary outcome measure agreed to by the FDA.

For more information about the REACH-CDM trial, visit https://www.clinicaltrials.gov/ct2/show/NCT03692312.

Our Focus on Phelan McDermid

As AMO Pharma continues to move forward with the pivotal REACH-CDM clinical trial, the company is also advancing promising research to find treatments for childhood-onset disorders associated with neurodevelopmental delay. In 2021, the company expects results will be announced from a Phase 2 study of AMO-01 in Phelan-McDermid patients who have also been diagnosed with epilepsy. The data will include insights on efficacy and safety, including duration of benefit. This research is currently being conducted at Mount Sinai School of Medicine in New York and Texas Children's Hospital in Houston. Recently, AMO Pharma Chief Scientific Officer Dr. Mike Snape provided an update on this program in interviews published in Drug Discovery News, Drug Development and Delivery and BioSpace. Following are some excerpts from his updates:

- A signaling pathway known as ERK that is found in cells throughout the body is known to be dysregulated in patients with many forms of solid tumors. As a result, this pathway has been a target in cancer research for many years.
- This same ERK pathway is also known to play an important role in neurodevelopment and could be a potential target in efforts to treat certain genetic encephalopathies, a series of rare or ultra-rare developmental disorders frequently associated with developmental delay, learning difficulties, autism spectrum symptoms and epilepsy.
- AMO-01 is an inhibitor of the Ras-ERK pathway. It is currently in development at AMO Pharma. AMO Pharma is supporting research to assess the potential benefits of treatment with AMO-01 in Phelan-McDermid Syndrome. For updates on when the results from this Phase 2 study will be available, check the AMO Pharma website at www.amo-pharma.com.

"We hope our work identifies and confirms new approaches in clinical research in the CNS space that can transform the sector and lead to improved quality of research and more targeted therapies designed to reduce disease burden by targeting the underlying biology in neurodevelopmental disorders."

Michael Snape, PhD
Chief Scientific Officer

Upcoming Events

Register now for **Myotonic Dystrophy Foundation's** Meet the DM Drug Developers: AMO Pharma webinar, taking place Friday, March 5, 2021 from 3:00PM – 4:00 PM EST. During this webinar, AMO Pharma leadership will share information about the REACH-CDM trial and answer questions from the DM1 community.

Join us for the **2021 MDA Virtual Clinical & Scientific Conference** taking place March 15 - 18, 2021. The conference will include concurrent sessions focusing on translational and preclinical research, clinical trials, and clinical management in NMD as well as current topics on COVID-19 and the impact on the NMD community. More information can be found here.

AMO PHARMA

www.amo-pharma.com

AMO Pharma, Ltd.
1 Park Row
Leeds LS1 5AB UK
+44 (0) 1483 319070

AMO Pharma, Ltd.
2608 Erwin Road
Suite 148, Box 212
Durham, NC 27705 USA

Find us on **LinkedIn**

For more information on the company, please email info@amo-pharma.com. If you are a patient or caregiver interested in matter(s) related to our pipeline or clinical trials, please email Medinfo@amo-pharma.com.