



Press Release

AMO Pharma Announces Preclinical Data Showing AMO-02 Improves Muscle Function, Glucose Handling and CNS function in Mouse Models of Duchenne Muscular Dystrophy

Sep 19, 2023

Findings from research collaboration with Brock University also show improved function in metabolic area and skeletal and cardiac muscle.

LONDON, Sept. 19, 2023 /PRNewswire/ -- AMO Pharma Limited ("AMO Pharma"), a privately held clinical-stage specialty biopharmaceutical company focusing on rare childhood-onset neurogenetic disorders with limited or no treatment options, today announced initial preclinical data from a study of the use of the company's investigational therapy AMO-02 in the treatment of Duchenne muscular dystrophy (DMD). In a mouse-model study conducted in collaboration with Brock University, treatment with AMO-02 was associated with improvements in muscle function and glucose handling as well as improvements in cardiac muscle function and CNS function.

In January 2023, Brock University and AMO Pharma announced initiation of a research partnership supported by a grant from the Brock-Niagara Validating, Prototyping and Manufacturing Institute (VPMI) to investigate the potential utility of GSK3 β inhibitors in the treatment of DMD, a rare genetic disorder characterized by progressive muscle degeneration and weakness. A team of researchers at Brock University led by professors Val Fajardo, PhD, and Rebecca MacPherson, PhD, investigated the effects of the GSK3 β inhibitor AMO-02 (tideglusib) and a follow-on compound in the *mdx.D2* mouse, a well characterized, naturally occurring murine model of DMD. Studies were conducted with one month of oral treatment initiated at either the start of the myodegenerative phase in mice (six to eight weeks) or at an advanced stage of disease progression (24 to 26 weeks). Assessments during treatment investigated the entire disease phenotype rather than being limited to solely muscle function and biomarker expression.

Initial studies showed that unlike chronic glucocorticoid treatment in *mdx* mice, which can acutely improve muscle function while dampening insulin sensitivity, AMO-02 treatment improved both muscle function and glucose handling and showed improved function in metabolic areas, muscle fat deposition and cardiac muscle. These benefits were seen in both early and late stage *mdx* mice. An additional study led by Dr. MacPherson examined the effects of AMO-02 treatment on cognitive function, as

pathology seen in disorders similar to Alzheimer's disease has been previously detected in *mdx* mice². These results showed that short-term treatment with AMO-02 significantly improved cognitive function.

"These data are important in several ways, particularly in showing the broad profile of efficacy in the *mdx* mouse at all stages of the degenerative process. Benefit was seen in multiple organ domains including heart and brain as well as in metabolic function, skeletal muscle, and glucose and fat handling," said Dr. Fajardo.

"These studies further reaffirm the strong potential of AMO-02 in treating the muscle damage and weakness that occurs with Duchenne muscular dystrophy and other muscle-wasting conditions, as well as the potential to improve cardiac and skeletal muscle health and function. We look forward to building a larger research collaboration with Brock and extend our gratitude to Drs. Fajardo and MacPherson and their teams for their outstanding work," said Michael Snape, PhD, AMO Pharma Chief Scientific Officer.

AMO Pharma also recently *announced results* from the REACH-CDM clinical study of *the investigational therapy AMO-02 in the treatment of children and adolescents with congenital myotonic dystrophy that showed statistically and clinically significant efficacy benefits in multiple areas including cognitive performance, reduction in biomarkers and improvements in walk/run measurements*. The company plans *to meet with regulators to outline a path forward for the development of AMO-02, including plans for study in patients with adult-onset myotonic dystrophy*.

About AMO Pharma

AMO Pharma is a clinical-stage specialty 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. 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, neither on the part of MHRA/Commission on Human Medicines (CHM) nor on the Company. Furthermore, advice cannot be taken as indicative of any future agreed position.

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

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