AMO Pharma Announces Initiation of Planned Pivotal Clinical Trial for Myotonic Dystrophy Following $35m Fund Raise

Trial to assess AMO-02 in treatment of congenital myotonic dystrophy to begin patient enrollment in early 2020.

LONDON DATE - 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 initiation of patient enrollment in the company's planned pivotal clinical trial for AMO-02 in the treatment of congenital myotonic dystrophy. This follows completion of discussions on the design and outcomes measures of the trial with regulators and AMO Pharma's successful execution of a $35m financing with new investors.

AMO Pharma's Chair, Ibraheem Mahmood said, "We are delighted to announce our new investment round brings $35m of new money in to the business. We can also confirm that shares previously managed by Woodford Investment Management have now moved to new fund managers with further details to follow."

"Based on this financing, AMO Pharma has the resources necessary to complete the AMO-02 clinical programme. We are working with our outstanding team of clinical investigators to advance this development program as rapidly as possible," said Dr Mike Snape, chief executive officer at AMO Pharma. "Congenital myotonic dystrophy is a devastating condition that represents a significant area of unmet need in global health with few and only inadequate and unapproved treatment options available. AMO-02 accesses brain, muscle and other tissues and reduces the DMPK expansion repeat mRNA that is the pathological basis for congenital myotonic dystrophy."

The AMO-02 clinical 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. The trial is being conducted at ten treatment centers across Canada, the US and the UK and plans to enroll a total of 56 patients. Patients will be assessed on a range of measures of CNS features and muscle function associated with congenital myotonic dystrophy.

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, debilitating childhood onset neurogenetic disorders with limited or no treatment options. As well as developing AMO-02 for congenital myotonic dystrophy, the company is 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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