AMO PHARMA

New medicines. Better lives.

A Unique Drug Development Model

AMO Pharma is identifying and advancing promising therapies based on a unique business model designed to achieve new levels of innovation and efficiency. Our approach is based on many core strengths:

- A comprehensive understanding of disease mechanism of action and impact on patient health and quality of life.
- A global network of contacts in research and drug development to identify de-risked therapies that present promising opportunities for clinical development.
- Insights to complete a comprehensive review of safety and efficacy data for each asset.
- Identification of clear protocols and opportunities to "jump start" and streamline clinical development programs.
- Flexibility to consider acquisition of assets to address unmet needs in a range of therapeutic areas and in smaller and larger patient populations.
- Established relationships with third parties related to advancing research, commercialization strategies, and partnering and licensing opportunities.

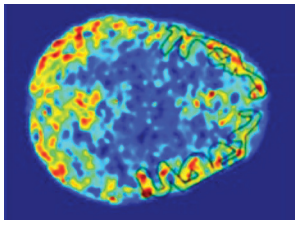
AMO Pharma, Ltd., is a privately held emerging biopharmaceutical company developing new treatments for serious and debilitating diseases including rare genetic disorders. Led by industry professionals with extensive experience in all phases of drug development and asset acquisition, the company is currently advancing clinical stage investigational products to treat neuromuscular or CNS symptoms of rare diseases. AMO Pharma plans to expand its portfolio to include additional promising therapies that represent important potential advances in patient care as well as strong commercial opportunities.

Pipeline

AMO Pharma's two lead investigational drugs currently in development, AMO-01 and AMO-02, are advancing into Phase 2 clinical trials.

	Preclinical	Phase 1	Phase 2	Phase 3
AMO-01 RAS-ERK inhibitor	*Fragile X Syndrome*			
AMO-02 GSK3β inhibitor	*Myotonic dystrophy*			

AMO-01 is in development for treatment of fragile X syndrome, the most common cause of autism and intellectual disabilities. The disorder is caused by a mutation in the FMR1 gene, and symptoms include social anxiety, hyperactivity and other autistic behaviors.



Dysregulated protein synthesis is thought to be a core phenotype of fragile X syndrome (FXS). In a mouse model (Fmr1 knockout (KO) of FXS, rates of cerebral protein synthesis are increased in selective brain regions. Protein synthesis can also be studied in human Fragile X patients using positron emission tomography.

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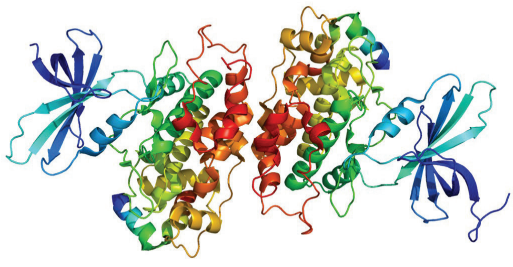
AMO-02 is in development for treatment of myotonic dystrophy, the most common form of muscular dystrophy (DM1). It is caused by a mutation in the DMPK gene and can result in increased levels of glycogen synthase kinase 3 beta (GSK3β) in patients. DM1 symptoms include muscle weakness and intellectual and developmental impairment.

The safety and efficacy profiles of these therapies and their demonstrated mechanisms of action provide strong support for their development in these indications, and also show that they have potential for use in the treatment of certain other orphan and non-orphan diseases.



A mutation in the DMPK gene leads to increased levels of glycogen synthase kinase 3 beta (GSK3ß) and the development of myotonic dystrophy (DM1).

By understanding the patient experience, we are better able to advance drugs that will reduce disease burden and give patients the best chance for improved quality of life and health

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AMO PHARMA

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Dedication to Patients

AMO Pharma's passion for drug development is strengthened and inspired by many personal connections to the rare disease community held by members of our senior team. As an essential element in our clinical development programs, we work to build connections with leaders in patient advocacy to understand all areas of unmet need and gain new insights related to the diseases we are targeting. These perspectives help us improve the quality of research and identify opportunities to expand disease awareness and patient education for the communities we serve. By understanding the patient experience, we are better able to advance drugs that will reduce disease burden and give patients the best chance for improved quality of life and health.

Management Team

Members of the AMO Pharma senior team have extensive experience in biomedical science and research with proven skill in all phases of drug development and commercialization. With bases in both the U.S. and the U.K., the company is able to leverage an international network of contacts and resources in all operational areas.

Michael Snape, PhD *Chief Executive Officer, Chief Scientific Officer*

Dr. Snape is a neurobiologist with extensive experience in the pharmaceutical industry, including leadership positions with large and emerging biopharmaceutical companies. He has founded and funded companies from inception through public offerings, and his principal experience is demonstrating clinical proof-of-concept with novel targeted therapeutics in rare diseases. He earned a PhD in neurobiology from London University and holds a faculty position at Case Western University.

Martyn Williams, MA, FCA *Chief Financial Officer*

Over the past 30 years Mr. Williams has held senior operational and financial management positions at several international businesses. Most recently he served as CEO of Ark Therapeutics plc. He has experience in international M&A transactions, including both acquisitions and divestments, as well as in strategic management of corporate growth and restructuring.

Joe Horrigan, MD *Chief Medical Officer*

Dr. Horrigan is a pediatric neuropsychiatrist. He was formerly Senior Director in the Neurosciences Medicines Development Center at GlaxoSmithKline and most recently served as Vice President of Clinical Development and Medical Affairs for Neuren Pharmaceuticals Limited. He received his undergraduate degree from Brown University and his medical degree from the University of Rochester.

Alison McMorn, PhD *Vice President, Clinical Development*

Dr. McMorn is an experienced clinical development researcher and has led, planned and managed global clinical development programs for gastro-intestinal/hepatology, pain and psychiatry products. Most recently she served as a Director of Clinical Programs at Shire Pharmaceuticals. She holds an undergraduate degree in Pharmacology and a PhD in psychology from the University of Leeds, UK.

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