ARMGO® Pharma Inc. Announces Clinical Trial of ARM210/S48168 for the Treatment of Ryanodine Receptor Type 1 Related Myopathies

ARDSLEY, N.Y., December 17, 2019 --ARMGO Pharma, Inc., a clinical stage biopharmaceutical company advancing a novel class of small molecule drugs known as Rycals®, announced today the start of a clinical trial using its Rycal ARM210 (also known as S48168), for the treatment for patients with Ryanodine Receptor Type 1 Related Myopathies (RYR1-RM). The trial is being performed in collaboration with the National Institute of Neurological Disorders and Stroke (NINDS) and the National Institute of Nursing Research (NINR) at the National Institutes of Health (NIH). In 2018, the FDA granted orphan drug designation to ARMGO for ARM210 as a potential treatment for patients with RYR1-RM.

ARM210 is a potential disease modifying therapy for genetic diseases, that targets the Ryanodine Receptor calcium channel (RyR), an intracellular calcium-release channel that becomes leaky in these and other diseases. Intracellular calcium leaks via mutant RyR1 channels impair muscle contraction leading to muscle weakness and loss of function, and activate toxic pathways that damage muscle, causing the symptoms in RYR1-RM.

ARM210 is a small molecule that binds to leaky RyR channels and repairs the leak, as demonstrated *in vitro* in muscle biopsies from RYR1-RM patients. The unique mechanism of action of ARM210 makes it an ideal potential disease modifying therapy for RYR1-RM. Muscle biopsies of the patients in this trial have been previously shown to respond biochemically to ARM210 *in vitro.* This trial will evaluate the safety and explore the biochemical effect of oral administration of ARM210 in these same patients.

"This trial represents an important achievement for the Rycal® ARM210 program as we hope to provide effective treatment for patients with RYR1-RM, for which there are no approved therapies," said Gene Marcantonio MD PhD, President and Chief Medical Officer of ARMGO Pharma. “We are looking forward to continuing the progress of the ARM210 clinical program, building on success in RYR1-RM

patients to address other genetic mutations of RYR 1 and 2." Further information can be found at https://clinicaltrials.gov/ Identifier: NCT04141670.

About ARMGO Pharma

ARMGO Pharma, Inc., is a privately held biopharmaceutical company dedicated to applying targeted mechanism-based science to the development of novel small-molecule therapeutics to treat cardiac, musculoskeletal, and neurological disorders characterized by leaky Ryanodine Receptor (RyR) calcium channels. Leaky RyRs are caused by genetic mutations as well as post-translational modifications of RyR channels. The company's proprietary drugs, known as Rycals®, are a new class of oral agents that repair calcium leaks through the RyR. ARMGO Pharma has been awarded an exclusive, worldwide license from Columbia University for its RyR technology based on the research of founding scientist Andrew R. Marks, M.D. The ARM210 program is supported through a Bench to Bedside grant from the NIH (NNS19005001), and a research collaboration and financial support from Les Laboratoires Servier.

For more information, please visit www.armgo.com

ARMGO Pharma Inc.
PMB #260, 923 Saw Mill River Road
Ardsley, NY 10502
info@armgo.com