ARMGO Pharma, Inc.
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ARMGO Pharma Receives FDA Orphan Drug Designation for ARM210/S48168 for the Treatment of Ryanodine Receptor Type 1 Related Myopathies

ARDSLEY, N.Y., September 5, 2018 --ARMGO Pharma, Inc., a clinical stage biopharmaceutical company advancing a novel class of small molecule drugs known as Rycals®, announced today that the U.S. Food and Drug Administration (FDA) has granted orphan drug designation to ARM210 (also known as S48168), a potential treatment for patients with Ryanodine Receptor Type 1 Related Myopathies (RYR1-RM). In 2015, the FDA granted orphan drug designation and rare pediatric disease designation to ARM210 as a potential treatment for Duchenne Muscular Dystrophy (DMD).

ARM210 targets the Ryanodine Receptor calcium-release channel (RyR), an intracellular calcium release channel that becomes leaky in a number of diseases including DMD and RYR1-RM, contributing to muscle damage and loss of function. RYR1-RM comprise a group of rare skeletal muscle diseases due to mutations in the *ryr1* gene, which lead to leaky channels. These leaks both 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 therapy for RYR1-RM.

"This designation represents an important achievement for the Rycal® ARM210 program and highlights the unmet need to provide effective treatment for patients with RYR1-RM," 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, including upcoming studies in RYR1-RM patients.”

About Orphan Drug Designation

Orphan Drug Designation is granted by the FDA to drug candidates intended to treat diseases affecting fewer than 200,000 patients in the U.S. Orphan designation qualifies the sponsor to apply for FDA orphan research grants, waiver of Prescription Drug User Fee Act filing fees, tax credits for clinical research costs, and a seven-year period of market exclusivity upon approval of the drug.

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. The company's proprietary drugs, known as Rycals®, are a new class of oral agents that repair the calcium leak through the RyR, which is located on the sarcoplasmic/endoplasmic reticulum. ARMGO Pharma has been awarded an exclusive, worldwide license from Columbia University for its RyR technology. Development and commercial rights for ARMGO's Rycal drugs in cardiovascular and skeletal muscle indications outside of the US and Japan are exclusively licensed to Les Laboratoires Servier (Servier). Development of ARM210 has been supported through a research collaboration with Servier, and an award from the Muscular Dystrophy Association (MDA USA).

For more information, please visit www.armgo.com

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