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ARMGO Pharma Receives FDA Orphan Drug Designation and Rare Pediatric Disease Designation for ARM210/S48168 for the Treatment of Duchenne Muscular Dystrophy

TARRYTOWN, N.Y., December 9, 2015 -- 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 and rare pediatric disease designation to ARM210 (also known as S48168), for the treatment for Duchenne Muscular Dystrophy (DMD), a devastating condition resulting in progressive and ultimately debilitating muscle weakness in young males.

ARM210/S48168 targets the Ryanodine Receptor (RyR), an intracellular calcium release channel that becomes leaky in disease states including DMD, contributing to muscle damage and loss of function. In animal models of DMD, ARM210 showed significant and robust improvements in exercise capacity, muscle specific force, grip strength and muscle histology compared to vehicle-treated controls. With its unique mechanism of action and oral delivery formulation, ARM210 has the potential to act across skeletal muscle, diaphragm and cardiac muscle regardless of genetic background, both as a mono-therapy as well as in conjunction with other treatments for DMD.

"These designations from FDA represent an important achievement for the ARM210 program and highlight the ongoing need to provide meaningful treatments for patients and families affected by Duchenne Muscular Dystrophy," said Dr. Sapan Shah, President and Chief Executive Officer of ARMGO Pharma. "We are looking forward to continuing our progress with the ARM210 clinical program, including completing ongoing Phase 1 clinical studies and advancing into Phase 2 studies in DMD patients."

ARMGO Pharma and its partner Servier previously announced the selection of ARM210/S48168 for advancement into the clinical stage following the successful completion of preclinical efficacy and

IND/CTA enabling GLP safety studies. A Phase 1 clinical study to assess the pharmacokinetics and safety of ARM210 in healthy male volunteers is currently underway in Europe. Following a successful conclusion of this study and completion of other enabling work including appropriate regulatory discussions, a Phase 2a study in DMD patients is planned.

About Orphan Drug Designation

Orphan Drug Designation is granted by the FDA to drug candidates intended to treat rare diseases or conditions affecting fewer than 200,000 patients in the U.S. Orphan designation qualifies the sponsor to be eligible for protocol assistance from FDA, the ability to apply for FDA orphan product research grants, waiver of Prescription Drug User Fee Act (PDUFA) filing fees, tax credits for clinical research costs, and a seven year period of market exclusivity upon approval of the drug.

About Rare Pediatric Disease Designation

The FDA defines a "rare pediatric disease" as a disease that affects fewer than 200,000 individuals in the U.S. primarily aged from birth to 18 years. Under the FDA's Rare Pediatric Disease Priority Review Voucher program, a sponsor who receives an approval of a new drug application (NDA) for a rare pediatric disease may be eligible for a voucher which can be redeemed to obtain priority review for any subsequent marketing application. The Priority Review Voucher may be sold or transferred an unlimited number of times.

About Duchenne Muscular Dystrophy

Duchenne Muscular Dystrophy (DMD) is a form of muscular dystrophy that is the most common X-linked inherited disorder in males affecting approximately 1 in 3,500 live male births with an estimated patient population exceeding 50,000 worldwide. DMD affects young males resulting in progressive and ultimately debilitating muscle weakness. Caused by mutations in the gene that makes dystrophin, a protein required for the normal structure and function of skeletal and cardiac muscles, DMD affects skeletal muscle force and function (ambulation and upper limb motion), diaphragm function (breathing) and cardiac function.

About ARMGO Pharma

ARMGO Pharma, Inc., is a privately held biopharmaceutical company dedicated to applying original, targeted science to the discovery and development of novel small-molecule therapeutics to treat debilitating cardiac, musculoskeletal, and neurological disorders, based on the research of founding scientist Dr. Andrew Marks. The company's proprietary drugs, known as Rycals®, are a new class of

oral agents that repair calcium leak through the ryanodine receptor calcium-release channel (RyR), which is located on the sarcoplasmic/endoplasmic reticulum of the cell. 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 have been exclusively licensed to Servier. Development of ARM210 has been supported through a research collaboration with Servier, along with an award from the Muscular Dystrophy Association (MDA USA).

For more information, please visit www.armgo.com

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