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ARMGO Pharma and Servier Announce Advancement of Rycal ARM210/S48168 into Clinical Stage Program Targeting Duchenne Muscular Dystrophy

TARRYTOWN, NY, and SURESNES, France, December 4, 2014 – ARMGO Pharma and Servier today announced the successful completion of preclinical efficacy and IND/CTA enabling studies with ARM210/S48168, along with a formal decision to advance the program into early clinical development initially targeting treatment for patients with Duchenne Muscular Dystrophy (DMD), the most common and severe form of muscular dystrophy.

ARMGO Pharma and Servier have collaborated since 2006 on the advancement of a novel class of small molecule drugs known as Rycals®, which were initially discovered through the pioneering work of Dr. Andrew R. Marks, the Clyde and Helen Wu Professor of Molecular Cardiology (in Medicine), Chairman of the Department of Physiology and Cellular Biophysics, and Founding Director of the Clyde and Helen Wu Center for Molecular Cardiology at Columbia University Medical Center. Rycals target the Ryanodine Receptor (RyR), an intracellular calcium release channel that becomes leaky in disease states including Duchenne Muscular Dystrophy, contributing to muscle damage and loss of function. Rycals have been shown in animal models of muscle disease to repair RyR-mediated intracellular calcium leak and thereby improve muscle specific force and exercise capacity. Further, Rycals have been shown to exert similar beneficial effects on cardiac muscle in animal models of heart disease. The proprietary drug candidate ARM210/S48168, discovered by ARMGO Pharma, was selected by the

ARMGO-Servier collaboration from a library of Rycal candidates for preclinical advancement as a potential treatment for DMD and other muscle disorders. With its unique mechanism of action and oral delivery formulation, ARM210/S48168 has the potential to provide benefit across skeletal muscle, diaphragm and heart muscle in DMD patients regardless of genetic background, both as a mono-therapy as well as in conjunction with other treatments.

In preclinical efficacy studies using the *mdx* mouse model of Duchenne Muscular Dystrophy, oral treatment with ARM210/S48168 showed significant and robust improvements in exercise capacity, specific muscle force, grip strength and muscle histology compared to vehicle-treated controls. These improvements were validated in both short term (4 week) and longer term (3 month) efficacy studies in *mdx* mice conducted by multiple, internationally recognized independent laboratories. Results from one of these studies were recently presented at the 19th World Muscle Society Congress, November 7-11, 2014 (Abstract G.P. 90, Capogrosso et al., Neuromuscular Disorders 24 (2014) 791-924). In addition to the preclinical efficacy models, an IND/CTA enabling package of GLP safety/toxicology studies with ARM210/S48168 has been completed by ARMGO and Servier, with no issues precluding further development.

Based on the collective strength of the completed preclinical efficacy and safety studies, ARM210/S48168 has been formally selected for advancement for clinical development by the ARMGO-Servier collaboration, resulting in a pre-agreed R&D payment by Servier to ARMGO, along with a commitment to support clinical development costs. Clinical studies will begin in 2015, following finalization of a global clinical development plan, completion of manufacturing and formulation work, and receipt of input from regulatory agencies. The initial therapeutic indication for the ARM210/S48168 clinical program is the treatment of Duchenne Muscular Dystrophy (DMD). Given the results of preclinical efficacy studies, ARM210/S48168 treatment has not only the potential to result in improvements in muscle function but also due to its unique mode of action, which repairs intracellular calcium leak in myopathy regardless of the causative mutation, to have an impact on the progression of the disease. Once efficacy is demonstrated in DMD patients, other forms of myopathy will be evaluated for advancement and may also benefit from this new treatment.

"We are very pleased to have formally reached this critical clinical advancement decision for ARM210 / S 48168 with our partner Servier," commented Dr. Sapan Shah, President and CEO of ARMGO Pharma. "We are excited at the prospect of bringing a novel therapy to patients suffering from skeletal muscle diseases such as DMD, where new treatments are desperately needed."

Dr. Emmanuel Canet, MD PhD, Vice-President Research and Development at Servier said, "This agreement demonstrates our long-term commitment in research to discover new therapy that will benefit young patients in a disease that has a major impact on the quality of life and life expectancy in the boys affected".

Dr. Patricia Belissa-Mathiot, Director of Innovative Center for Rheumatology at Servier stated, "Given the unique mode of action of ARM210/S48168, which corrects intracellular calcium leak regardless of the underlying genetic mutation, its therapeutic value could be assessed in other myopathies once efficacy is established in DMD."

About DMD

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 function (decrease exercise capacity), 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. 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 Les Laboratoires Servier (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 further information visit: www.armgo.com

About Servier

Servier is an independent French pharmaceutical research company. Its development is based on the continuous pursuit of innovation in the therapeutic areas of cardiovascular-, metabolic-neurologic-, psychiatric-, bone- and joint diseases as well as cancer.
In 2013, the company recorded a turnover of 4.2 billion euros.
91 percent of Servier drugs are consumed outside France.
27 percent of turnover from Servier drugs were reinvested in Research and Development in 2013.
With a strong international presence in 140 countries, Servier employs more than 21,000 people worldwide.

More information: www.servier.com