



**Contact:**

Dr. Sapan Shah  
CEO, ARMGO Pharma  
(914) 425-0000  
[info@armgo.com](mailto:info@armgo.com)

Roxan Olivas  
MDA Vice President — Public Relations  
(520) 529-5305  
[rolivas@mdausa.org](mailto:rolivas@mdausa.org)

ARMGO Pharma Receives \$1 Million Award from MDA to Support Advancement of Rycal Compound ARM210 as a Novel Treatment for Duchenne Muscular Dystrophy

**TARRYTOWN, N.Y. and TUCSON, Ariz., April 17, 2013** – ARMGO Pharma and the Muscular Dystrophy Association ([MDA](#)) today announced that \$1 Million has been awarded for preclinical work in support of an Investigational New Drug (IND) application with the U.S. Food and Drug Administration (FDA) for ARM210, a novel, orally available, small-molecule Rycal drug that has potential as a treatment for Duchenne muscular dystrophy (DMD). The funding award to ARMGO Pharma comes from [MDA Venture Philanthropy \(MVP\)](#), part of MDA's translational research program.

The project being advanced by ARMGO Pharma is focused on a novel class of small molecule drugs known as Rycals™ which target the Ryanodine Receptor (RyR), an intracellular calcium channel that becomes leaky in disease states including muscular dystrophy, contributing to loss of muscle strength and function. Rycals have been shown in animal models of muscle disease to repair RyR-mediated calcium leak and thereby improve specific muscle force and exercise capacity. ARMGO Pharma's experimental proprietary drug ARM210 has been selected by the company from its library of Rycal candidates for preclinical advancement as a potential treatment for DMD. With its unique mechanism of action and oral delivery formulation, ARM210 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.

[DMD](#) is one of nine types of muscular dystrophy, a group of genetic, degenerative diseases primarily affecting voluntary muscles. Caused by mutations in the gene that makes dystrophin, a protein that normally protects muscle cells and keeps them intact, DMD eventually weakens all voluntary muscles, and the heart and breathing muscles. DMD affects approximately 1 in 3,500 boys with an estimated patient population exceeding 50,000 worldwide.

"We are very pleased to have been selected by the MDA to receive funding which will support the advancement of ARM210" said Dr. Sapan Shah, President and CEO of ARMGO Pharma. "We are excited at the possibility to expand our Rycal program into skeletal muscle diseases such as DMD, where new treatment options for patients are desperately needed."

"This is a very interesting potential therapeutic" said Jane Larkindale, MDA's vice president for research. "It works in a different way than the other potential therapeutics in development, which suggests that in addition to being effective on its own, it also might be able to be added on to other treatments, when other treatments become available."

### **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<sup>TM</sup>, 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. ARMGO Pharma's lead Rycal program ARM036 is in Phase 2 clinical studies for the treatment of heart failure and arrhythmias. 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).

### **About MVP**

[MDA Venture Philanthropy](#) program, the drug development arm of the Association's translational research program, made the award to ARMGO. The grant will fund laboratory work

which ARMGO must complete before the biotech can submit an investigational new drug (IND) application to the U.S. Food and Drug Administration. An IND application is required by the FDA before a new drug can move to clinical trials in humans.

### **About MDA**

MDA is the nonprofit health agency dedicated to finding treatments and cures for muscular dystrophy, ALS and related diseases by funding worldwide research. The Association also provides comprehensive health care and support services, advocacy and education.

In addition to funding more than 250 research projects worldwide, MDA maintains a national network of 200 medical clinics; facilitates hundreds of support groups for families affected by neuromuscular diseases; and provides local summer camp opportunities for thousands of youngsters living with progressive muscle diseases.

For more information, visit [mda.org](http://mda.org) and follow MDA on Facebook ([facebook.com/MDAnational](https://www.facebook.com/MDAnational)) and Twitter ([@MDAnews](https://twitter.com/MDAnews)).

SOURCE: ARMGO Pharma and Muscular Dystrophy Association

### RELATED LINKS

<http://www.armgo.com>

<http://www.mda.org>