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## **ARMGO Pharma Receives Research Grant Award from Kennedy's Disease Association to Support Advancement of Rycal Compounds**

**TARRYTOWN, NY and COARSEGOLD, CA, November 6, 2013** – ARMGO Pharma and the Kennedy's Disease Association (KDA) today announced that a research grant has been awarded to support preclinical work on novel, orally available compounds that have potential as a novel treatment for Kennedy's Disease (also known as Spinal Bulbar Muscular Atrophy or SBMA). The project being advanced by ARMGO Pharma is focused on a novel class of small molecule drugs known as Rycals<sup>®</sup> which target the Ryanodine Receptor (RyR), an intracellular calcium channel that becomes leaky in disease states, including Kennedy's Disease, and contributes to loss of muscle strength and function.

Kennedy's Disease (KD) is a rare and currently incurable and non-treatable recessive X-linked, genetic progressive neuromuscular disease. Spinal and bulbar motor neurons are primarily affected causing muscle weakness and wasting (atrophy) throughout the body including the extremities (legs/arms) as well as in the face and throat. This results in speech and swallowing difficulties as well as major motor deficiencies. Kennedy's Disease is an adult-onset disease with symptoms usually appearing between the ages of 30 and 50. It is estimated that 1 in 40,000 male individuals worldwide have the disease; however many go undiagnosed or misdiagnosed for years. The most frequent misdiagnosis is the fatal Lou Gehrig's Disease (ALS).

The focus of the research project is to assess the therapeutic effects of orally bioavailable, small molecule modulators of the RyR (known as Rycals) which repair pathophysiologic calcium leak in skeletal muscle disease to reverse chronic muscle weakness. Given recent evidence that Kennedy's Disease may affect muscles directly, causing them to become weak independent of the nervous system, such modulators have the potential to improve motor function in patients affected by KD (SBMA). Rycals restore normal RyR function, improve skeletal muscle biochemical markers and improve function of pathologic muscle in various preclinical models of skeletal muscle disease. Recent evidence from a mouse model of KD suggests that toxic androgen receptor may cause muscle weakness by impairing the function of RyR, resulting in an elevated concentration of cytosolic calcium and the loss of muscle strength in KD affected men.

"We are very pleased to have been selected by the KDA to receive funding which will support the advancement of our scientific platform against Kennedy's Disease, complementing our lead Rycal programs for heart failure and Duchenne Muscular Dystrophy" said Dr. Sapan Shah, President and CEO of ARMGO Pharma. "We are excited at the possibility to expand our Rycal research and development program into skeletal muscle diseases, such as Kennedy's Disease, where new treatment options for patients are desperately needed" remarked Dr. Michael Blonar, Chief Scientific Officer of ARMGO Pharma and Principal Investigator of the KDA-funded research project.

Upon granting the award, the Kennedy's Disease Association commented, "The KDA is pleased to be able to partner with ARMGO, as part of our overall effort to cure KD. Their proposal was highly ranked by our scientific reviewers and we are hopeful that this unique approach to combatting KD will lead to a treatment in the near future."

**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>®</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.

**About the Kennedy's Disease Association:** The Kennedy's Disease Association is an all-volunteer organization, with 91¢ of every dollar funding Kennedy's Disease Research Grants and education. The Mission of the Kennedy's Disease Association is to:

1. Financially support and promote medical research to find a cure for Kennedy's Disease
2. Share information about Kennedy's Disease for those who seek it
3. Improve awareness of Kennedy's Disease in the medical community
4. Create a support system for those living with Kennedy's Disease
5. Increase public awareness about Kennedy's Disease and its effects on families

Kennedy's Disease Association, P.O. Box 1105, Coarsegold, CA 93614-1105

SOURCE: ARMGO Pharma and the Kennedy's Disease Association

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