

RyCarma Announces First Patient Dosed in Phase 2 Clinical Trial of Surlorian for the Treatment of RYR1-Related Myopathy

- *RYR1-RM is one of the most common congenital skeletal muscle disorders and has no approved therapeutics*
- *Surlorian (ARM210) is a novel small molecule therapeutic designed to restore skeletal muscle function by targeting ryanodine receptors and calcium leak*
- *Phase 2 topline data are anticipated in the second half of 2027*

BOSTON--([BUSINESS WIRE](#))--RyCarma Therapeutics, Inc., a clinical-stage biotechnology company developing first-in-class small molecule therapeutics for skeletal muscle diseases, today announced that the first patient was dosed in a Phase 2 clinical trial of surlorian in patients with RYR1-related myopathy (RYR1-RM).

RYR1-RM is caused by a mutation in the RYR1 gene and is one of the most prevalent congenital skeletal muscle disorders. The disease is characterized by impaired muscle contraction, weakness, and significant loss of independence, as well as other serious symptoms that affect quality of life. Approximately 80% of people with RYR1-RM experience difficulty with basic ambulation, and many require a wheelchair. There are no approved therapeutics for RYR1-RM; the current standard of care for patients relies on managing symptoms and attempting to maintain independence through physical therapy, respiratory support, and energy conservation.

Surlorian is RyCarma's lead molecule in development, representing a novel class of therapeutics designed to restore skeletal muscle function by targeting ryanodine receptors and calcium leak. In Phase 1 clinical studies in healthy participants and in RYR1-RM patients, surlorian demonstrated favorable safety, tolerability, and pharmacokinetics. In a Phase 1b open-label trial (NCT04141670) exploring two different doses of surlorian for 28 days, RYR1-RM patients on the higher dose reported increased proximal muscle strength as well as reduced fatigue (as measured by PROMIS score), a key symptom of RYR1-RM. Surlorian has received Orphan Drug and Rare Pediatric Disease Designations from the FDA and is the only known therapeutic candidate currently in clinical trials for RYR1-RM.

"There are currently no approved treatments for RYR1-RM, leaving individuals and families with limited therapeutic options for a condition that can profoundly affect daily life and independence. Encouraging advances in our understanding of RYR1-RM are paving the way for therapies that target the underlying disease mechanisms," said Lindsay Goldberg, Executive Director of The RYR-1 Foundation. "If proven safe and effective, a treatment like surlorian, which targets calcium leak through the ryanodine receptor, could represent an important step forward for the RYR1-RM community."



The Phase 2, randomized, double-blind, placebo-controlled, multicenter study (NCT07560020, EU CT 2025-522343-18-00) will involve approximately 28 patients with autosomal dominant RYR1-RM and proximal muscle weakness. Each patient will receive 28 days of surlorian and 28 days of placebo in randomized sequence, separated by a 21-day washout period. Following the double-blind portion of the study, patients will have the option to enroll in an open-label extension and receive continued treatment with surlorian for up to 12 months. The trial's primary endpoint is change from baseline in the 1-minute sit-to-stand test; secondary endpoints include a variety of functional performance measures, strength testing, and patient-reported outcomes. The topline readout is expected in the second half of 2027.

“With surlorian, RyCarma has the opportunity to bring a first-in-class therapy to patients with skeletal muscle disorders who lack suitable treatment options,” said Adam Rosenberg, Chief Executive Officer of RyCarma. “In addition to our Phase 2 study in RYR1-RM, which is now enrolling patients at leading RYR1-RM investigator sites, RyCarma also has an open IND to evaluate surlorian in Duchenne Muscular Dystrophy patients. With a strong group of investors and experienced drug development team, we are well positioned to advance surlorian through clinical studies and closer to patients.”

About RYR1-RM

RYR1-Related Myopathy is a rare skeletal muscle disease that is caused by mutations in the RYR1 gene, which encodes a calcium release channel located in the sarcoplasmic reticulum of skeletal muscle cells. Disease-causing mutations destabilize the channel, causing calcium ions to leak from intracellular stores into the cytoplasm. This disruption of calcium homeostasis impairs muscle contraction, damages muscle cells, and leads to muscle weakness.

About RyCarma Therapeutics

RyCarma is a clinical-stage biotechnology company developing first-in-class small molecule therapeutics for skeletal muscle diseases. The company is conducting a Phase 2 trial in RYR1-RM and is planning a clinical study in patients with Duchenne Muscular Dystrophy. RyCarma's drug discovery platform is built from deep expertise on ryanodine receptors (RyRs), channels that regulate the intracellular flow of calcium ions in nearly all cell types. RyCarma's RyR technology is based on the groundbreaking research of the company's founder, Dr. Andrew Marks. For more information, visit www.rycarma.com.

Contacts

Investors:

investors@rycarma.com

Media:

Ten Bridge Communications

TBCrycarma@tenbridgecommunications.com