

**2025 RYR-1-RELATED DISEASES PATIENT-LED INTERNATIONAL RESEARCH WORKSHOP:
NOVEL PERSPECTIVES, TREATMENTS & INTERVENTIONS**

Topic/Session	Session Chair/Confirmed Speakers
Session: Diagnosis & Genetics of RYR-1-RD	Livija Medne, MS, LCGC
RYR1 Database	Joshua Todd, PhD & Tokunbor Lawal, PhD
ClinVar VCEP Efforts for RYR1 Variant Curation	TBD
Functional Readouts	TBD
Session: Clinical Trial Readiness	Payam Mohassel, MD
Natural History and Registries	Nicol Voermans, MD, PhD
Muscle Imaging	TBD
Wearables	Ashkan Vaziri, PhD
Session: Novel Insight into RyR1 Interplay and Cell Pathophysiology	Johanna Lanner, PhD
Ion and Small Molecule Interplay with RYR1 Function	Montserrat Samso, PhD
Reduced RyR1 Content and Cellular Stress	Nadège Zanou, MD, PhD
MH and Altered Glucose Handling in Muscle	Eshwar Tammineni, PhD
Session: RYR1 and Knowledge Gained from Animal Models	Johanna Lanner, PhD
From Zebrafish to Rodents	TBD
Dog Model	TBD
Neuromuscular Regeneration in Organoids	Francesco Saverio Tedesco, MD, PhD
Session: Therapeutic Pipeline	Michael Goldberg, MD, MPH
Gene Editing and Gene Replacement Therapy for RYR-1-RD: Opportunities and Challenges	Isabelle Marty, PhD
Novel Gene Transfer Approaches: Split Inteins	Hichem Tasfaout, PhD
Muscle Regeneration	Nicolas Dumont, PT, PhD
Session: Physical Therapy & Exercise Approaches in RYR-1-RD	Mairéad Ryan, BS, V27
Physical Therapy General Guidelines for RYR-1-RD	Minal Jain, PT, MS, DSc
Exercise: Strength vs Cardiovascular & Muscle Function	Johanna Lanner, PhD