

1. Dezember 2025

**Meine lieben Freundinnen und Freunde,**

On behalf of our team at Boston Children's Hospital, I would like to thank you for your generous support of our work on *SELENON*-related myopathy (*SELENON*-RM). Your gift is helping to drive progress in our efforts to better understand and treat this rare and complex muscle disease. With your support, our team is advancing drug-screening techniques, improving a zebrafish model to test therapies, and creating a novel gene therapy.

For example, Dr. Pamela Barraza-Flores, one of our postdoctoral fellows, recently published a study demonstrating how the *SELENON* gene is important for muscle growth and function. Using both zebrafish and cellular models of *SELENON*-RM, this work showed that *SELENON* is essential for early muscle development and for maintaining healthy energy sources in muscle cells (mitochondria). This study adds valuable information to the understanding of muscle biology and possible treatment methods.

Our team also recently presented on new gene therapy research at the World Muscle Society conference in Prague. We developed a faster method to assess gene therapy effects in damaged muscle and engineered a new version of the *SELENON* gene for therapeutic testing. While this initial approach increased a key muscle protein, it did not yet fully restore muscle function due to limitations in reaching the correct cells. We are now actively working on an improved version designed to overcome this challenge. Thanks to the work of our colleague Dr. Jeffrey Widrick, our team can now identify muscle abnormalities in *SELENON* mouse models earlier, which accelerates our testing and progress.

We are deeply grateful for your continued support. Your generosity is enabling these advances and helping us move closer to treatments for individuals and families affected by *SELENON*-RM.

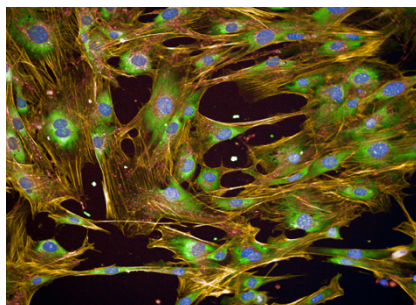
Mit herzlichen Grüßen



Alan H. Beggs, PhD  
Professor of Pediatrics, Harvard Medical School  
Director, Manton Center for Orphan Disease Research  
Boston Children's Hospital



Beggs Laboratory Research Team, 2025



*SELENON* Cell Painting Assay

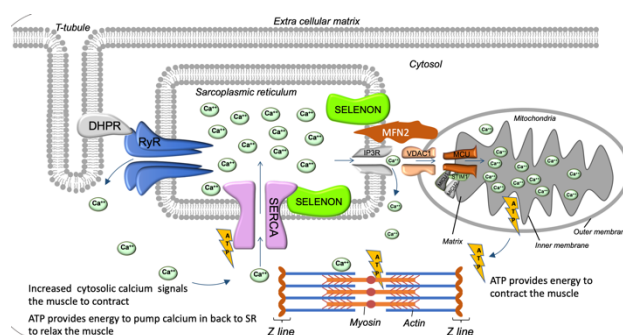


Diagram of *SELENON* function in muscle