
Advancing Neuromuscular Drug Development Through Patient-Centered Collaboration and Data Integration

The newest episode of the Critical Path Institute Podcast explores the essential collaboration between patients, researchers, regulators and drug developers working to accelerate drug development for neuromuscular diseases, particularly Duchenne muscular dystrophy and other muscular dystrophies. In honor of World Duchenne Day on September 7, this timely discussion features Ramona Belfiore-Oshan, Executive Director of C-Path's Duchenne Regulatory Science Consortium, and Pat Moeschen, a patient advocate with Parent Project Muscular Dystrophy who is living with limb-girdle muscular dystrophy. They join C-Path CEO and podcast host Klaus Romero to highlight how patient voices have evolved from passive observers to active partners in clinical trial design, endpoint selection and drug approval processes.

Key Insights

- **A Model of Collaborative Efficiency:** The Duchenne Regulatory Science Consortium was founded on the premise of removing data silos between researchers, regulators, industry and patients. This open collaboration accelerates scientific discovery and regulatory acceptance by pooling natural history data, placebo arm data and biomarker research to create robust disease progression and trial simulation models.
 - **Lived Experience Improves Relevance:** while historically the patient community was a passive recipient of drug development decisions the paradigm has now shifted. This community now helps shape clinical trial endpoints and trial design, ensuring that what is measured reflects meaningful changes in daily life.
 - **Heterogeneity Challenges Endpoint Selection:** Muscular dystrophies present varied genetic subtypes and highly individualized progression. This necessitates a nuanced understanding that no single endpoint fits all. For clinical trials, endpoints must consider abilities that matter the most, at each disease stage (e.g., ambulation in early stages, hand strength in later stages).
 - **Integrating Lived Experience into Rigorous Drug Development Tools:** The consortium's approach includes evaluating velocity measures, imaging biomarkers and fluid biomarkers to find objective, reliable tools reflecting disease progression. Simultaneously, patient advocate and caregiver input shapes understanding of fatigue, trial burden, and practical feasibility to reduce trial barriers and improve outcome measurement reliability.
- Communication and Connection as Catalysts for Progress:** Pat emphasizes that the power of networks — linking advocacy groups, researchers, regulators, and industry — is crucial. Communication creates opportunities for patients' voices to reach decision-makers and fosters hope within the community.

This episode demonstrates the vital synergy between the scientific rigor of model-driven drug development and the lived experiences of patients advocating for meaningful outcomes, illustrating a collaborative pathway towards transformative therapies in rare neuromuscular diseases.

Listen now:

