**Agenda**

8:30 a.m. **Registration**

9:00 a.m. **Welcome**
Ronald Bartek — Friedreich’s Ataxia Research Alliance (FARA)
Annie Kennedy — Parent Project Muscular Dystrophy (PPMD)

9:15 a.m. **Introduction and Demonstration of the Toolkit Portal**
Petra Kaufmann, MD — NCATS

10:00 a.m. **Break** (15 minutes)

10:15 a.m. **Case Study #1**
Megan O’Boyle — Phelan-McDermid Syndrome Foundation
Discovery and Pre-Clinical Case Study: Developing a Registry for Phelan-McDermid Syndrome

10:45 a.m. **Case Study #2**
Jennifer Farmer, MS, CGC — FARA
Clinical Trial Readiness Case Study: Friedreich’s Ataxia Experience

11:15 a.m. **Case Study #3**
Kenneth Hobby — Cure SMA
Translating Pre-Clinical to Clinical Case Study: Spinal Muscular Atrophy Experience

11:45 a.m. **Lunch** (1 hour)

12:45 p.m. **Case Study #4**
Annie Kennedy — PPMD
Post-Approval Activities Case Study: Duchenne Muscular Dystrophy Experience

1:15 p.m. **Discussion Panel**
Megan O’Boyle, Jennifer Farmer, Kenneth Hobby, and Annie Kennedy

2:15 p.m. **Break** (15 minutes)

2:30 p.m. **Next Steps for the Toolkit**
Group discussion (all participants)
Moderated by Ron Bartek, Annie Kennedy, Chuck Mohan, and Michelle Campbell (Food and Drug Administration)

3:30 p.m. **Closing Remarks**
Ron Bartek, Annie Kennedy, Anne Pariser (NCATS)

4:00 p.m. **Adjourn**