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
*Expected Content:* Discovery and Pre-Clinical Case Study: Developing a Registry for Phelan-McDermid Syndrome

11:00 a.m.  Case Study #2
Jennifer Farmer, MS, CGC
FARA
*Expected Content:* Clinical Trial Readiness Case Study: Friedreich’s Ataxia Experience

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

12:45 p.m.  Case Study #3
Kenneth Hobby
Cure SMA
*Expected Content:* Translating Pre-Clinical to Clinical Case Study: Spinal Muscular Atrophy Experience

1:30 p.m.  Case Study #4
Annie Kennedy
Parent Project Muscular Dystrophy
*Expected Content:* Post-Approval Activities Case Study: Duchenne Muscular Dystrophy Experience

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

2:30 p.m.  Next Steps for the Toolkit

3:30 p.m.  Closing Remarks

4:00 p.m.  Adjourn