



M-CERSI Conference on Patient- Focused Drug Development

March 9, 2015

Bethesda, MD



Planning Committee

AstraZeneca	Kathy Gans-Brangs
Avalere Health	Tanisha Carino
CEOi	Drew Holzapfel
Critical Path Institute	Stephen Coons
Epstein Health	Robert Epstein
FDA	Theresa Mullin, Ashley Slagle, Sara Eggers, Pujita Vaidya
Kaiser Permanente	Murray Ross
Lora Group	Laurie Burke
M-CERSI	James Polli and Ann Anonsen
National Health Council	Marc Boutin
National Organization for Rare Disorders	Peter Saltonstall
National PKU Alliance	Christine Brown
National Quality Forum	Karen Johnson
Novartis	Dr. Gretchen Trout
PatientsLikeMe	Sally Okun
Parent Project Muscular Dystrophy	Pat Furlong
Pfizer	Roz Schneider
PhRMA	Kristin Van Goor
PROEM	Eleanor Perfetto, Elisabeth Oehrlein, and Chinenye Anyanwu
Sanofi	Anne Beal

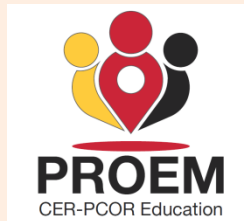


Thank You to Our Supporters



Inspired by **patients**.
Driven by **science**.

Thank You to Our Collaborators



LORA Group, LLC



**Parent Project
Muscular Dystrophy**
LEADING THE FIGHT TO END DUCHENNE

patientslikeme®



Conference Objectives

- Definition(s) of PFDD
- Essential components that need to be included in successful PFDD
- Current initiatives, such as those of the FDA, patient advocacy groups, and pharmaceutical companies
- Challenges to achieving PFDD and methods to overcome those challenges
- Recommendations on a plan of action that includes a description of stakeholders' roles to move PFDD forward



Conference Deliverables

- Recorded Conference
- Patient-Focused Drug Development Rubric
- Brief Report