Community Engagement to Determine Acceptable Benefits/Risks of Emerging Therapies

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Case Study:
Parent Project Muscular Dystrophy’s FDA Advocacy

• Followed FDA legislation introducing Patient-Focused Drug Development
• Identified compelling need to inform FDA about Duchenne/Becker muscular dystrophy community preferences and priorities for therapeutic dev’t
• Developed pilot research study on benefit/risk preferences as part of a comprehensive engagement effort with the FDA
Case Study: PPMD Con’t

• Benefit-risk preference data jump-started FDA engagement
• Comprehensive FDA outreach effort led to community-engaged development of draft guidance for DMD therapeutic dev’t
• Subsequent patient/caregiver preference studies (ongoing):
  – Therapeutic-specific preference study to assess perception of benefit and maximum acceptable risk
  – Preference study evaluating meaningful benefits and tolerance for uncertainty

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Importance of Measuring Preferences

• The FDA:
  – Wants to improve their benefit-risk framework
  – Is mandated to better understand the patient experience and preferences
  – Is interested in your testimony but... deals in data
Do Statistical Approaches Replace Testimony?

“I understand the need for caution and care, but I also know that our children are dying. Parents should be able to decide the risk/benefit of a drug that has gone through and passed preliminary testing. I would rather my son die trying and fighting than waiting and wondering and wishing....I am one parent willing to take an educated risk!” [PPMD Share Your Story]
Demands and Benefits of Leading a Preference Study to a Disorder Community

• Highly collaborative process
• Outcomes inform advocacy
• A large number of people can participate, even those who cannot/do not want to testify

• Resource and time intensive
• You may not get the results you “want”
• Requires larger sample sizes; more generalizable than testimony
Your Community’s Role...

Collaborating with experts in stated preference research to:

• Set the research agenda
• Develop a meaningful study
• Weigh in on interpreting the results
• Disseminate to sponsors and regulators (and your community)
Learn More:

• Advocacy-led preference research model:
  http://www.clinicaltherapeutics.com/article/S0149-2918(14)00209-4/pdf

• PPMD’s FDA engagement:
  http://www.parentprojectmd.org/site/PageServer?pagename=Advocate_fdaguidance

• FasterCures benefit-risk assessment tools: