Welcome and Introductions
Mark McClellan, Duke-Margolis Center for Health Policy

Opening Remarks
Laura Lee Johnson, U.S. Food and Drug Administration

Session I: Using Prior Data from Early Phase Trials to Inform Phase 3 Designs
Moderator: Mark McClellan

Presentation: Bayesian Borrowing of Historical Data for Confirmatory Clinical Trials
Karen Price, Eli Lilly and Company
Presentation: Incorporating Historical Controls in Phase 3 Designs
John Scott, U.S. Food and Drug Administration
Lead Reactant
Lisa LaVange, University of North Carolina at Chapel Hill
Panel Discussion
Lucas Kempf, U.S. Food and Drug Administration
Gigi McMillan, Bioethics Institute at Loyola Marymount University
Gary Rosner, Johns Hopkins University
Roy Tamura, University of Southern Florida
Audience Q&A

Break

Session II: Utilizing Patient Registry and Natural History Study Data to Advance Therapeutic Development for Rare Diseases
Moderator: Mark McClellan

Presentation: Utilizing Patient Registry and Natural History Study Data to Advance Therapeutic Development for Rare Diseases
Nicole Mayer-Hamblett, University of Washington
Lead Reactant
Patroula Smpokou, U.S. Food and Drug Administration
Panel Discussion
Randall Bateman, Washington University in St. Louis
Yeh-Fong Chen, U.S. Food and Drug Administration
Petra Kaufman, AveXis
PK Tandon, Ultragenyx Pharmaceutical
Audience Q&A
12:15 p.m.  Lunch

1:15 p.m.  Session III: Leveraging Master Protocols for Trials with Small Patient Populations
Moderator: Gregory Daniel, Duke-Margolis Center for Health Policy

Presentation: Master protocols in Rare Diseases: The Potential and the Challenges
Scott Berry, Berry Consultants
Lead Reactant
Michael Proschan, National Institute of Allergy and Infectious Diseases
Panel Discussion
Billy Dunn, U.S. Food and Drug Administration
Rajeshwari Sridhara, U.S. Food and Drug Administration
Audience Q&A

2:30 p.m.  Opportunity for Public Comment
Moderator: Mark McClellan

3:15 p.m.  Break

3:30 p.m.  Synthesis Discussion: Key Themes and Takeaways
Moderator: Gregory Daniel

Panel Discussion
Julie Beitz, U.S. Food and Drug Administration
Abby Bronson, Parent Project Muscular Dystrophy
Aloka Chakravarty, U.S. Food and Drug Administration
Cartier Esham, Biotechnology Innovation Organization
Telba Irony, U.S. Food and Drug Administration
Lisa LaVange, University of North Carolina at Chapel Hill
Rich Moscicki, Pharmaceutical Research and Manufacturers of America
Jerry Schindler, Harvard University
Ellen Werner, National Institutes of Health
Issam Zineh, U.S. Food and Drug Administration

4:45 p.m.  Meeting Summary and Priority Next Steps
Laura Lee Johnson

4:55 p.m.  Closing Remarks
Gregory Daniel

5:00 p.m.  Adjournment

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