Duke | Robert J. Margolis, MD Center for Health Policy

Public Meeting: Utilizing Innovative Statistical Methods and Trial Designs in Rare Disease Settings

DoubleTree by Hilton • Silver Spring
March 19, 2018

9:00 a.m. **Welcome and Introductions**
Mark McClellan, Duke-Margolis Center for Health Policy

9:05 a.m. **Opening Remarks**
Laura Lee Johnson, U.S. Food and Drug Administration

9:20 a.m. **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

10:50 a.m. **Break**

11:00 a.m. **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**

Funding for this workshop was made possible by the Food and Drug Administration through grant 5U19FD004971. Views expressed in the written materials and by speakers and moderators do not necessarily reflect the official policies of the Department of Health and Human Services nor does any mention of trade names, commercial practices, or organization imply endorsement by the United States Government.