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

Think Tank: Exploring Novel Trial Designs and Innovative Statistical Tools in Rare Disease Drug Development

Duke-Margolis Center for Health Policy • Washington, DC
May 3, 2017

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

9:15 a.m. **Opening Remarks**
Lisa LaVange and Rich Moscicki, U.S. Food and Drug Administration

9:30 a.m. **Session I: Using Prior Data from Early Phase Trials to Inform Phase 3 Designs**

Presentation: *Karen Price, Eli Lilly & Company*
Reactant: *John Scott, U.S. Food and Drug Administration*
Moderated discussion

10:45 a.m. **Break**

11:00 a.m. **Session II: Utilizing External Control Groups in Single Arm Trials**

Presentation: *Lilly Yue, U.S. Food and Drug Administration*
Reactant: *Nicole Mayer-Hamblett, University of Washington*
Moderated discussion

12:15 p.m. **Lunch**

1:00 p.m. **Session III: Leveraging Innovative Adaptive Trial Designs**

Presentation: *Yeh-Fong Chen, U.S. Food and Drug Administration & Roy Tamura, University of South Florida*
Reactant: *Olga Marchenko, QuintilesIMS*
Moderated discussion

2:15 p.m. **Session IV: Developing Analytical Techniques for Trials with Multiple Endpoints**

Presentation: *Laura Lee Johnson, U.S. Food and Drug Administration*
Reactant: *P.K. Tandon, Ultragenyx*
Moderated discussion

3:30 p.m. **Break**

3:40 p.m. **Session V: Advancing Development and Use of Promising Approaches**

Roundtable discussion

4:40 p.m. **Meeting Summary and Priority Next Steps**
Lisa LaVange, U.S. Food and Drug Administration

4:55 p.m. **Closing Remarks**
Greg Daniel, Duke-Margolis Center for Health Policy

5:00 p.m. **Adjournment**

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