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

Leveraging Randomized Clinical Trials to Generate Real-World Evidence for Regulatory Purposes

The Westin Washington, DC City Center
1400 M St NW • Washington, DC 20005
July 11, 2019

Agenda

8:30 a.m. **Registration**

9:00 a.m. **Welcome and Overview**
Gregory Daniel, Duke-Robert J. Margolis, MD, Center for Health Policy

9:05 a.m. **FDA Opening Remarks**
Jacqueline Corrigan-Curay, U.S. Food and Drug Administration

9:15 a.m. **Leveraging Randomized Designs to Generate RWE**
Presenters:
- **Robert Temple,** U.S. Food and Drug Administration
- **Lesley Curtis,** Duke University

9:45 a.m. **Selecting Interventions and Study Designs to Generate RWE**
Moderator: **Gregory Daniel**
Presenters:
- **Martin Landray,** Oxford University
- **Elaine Irving,** GlaxoSmithKline

Panelists:
- **Iris Goetz,** Eli Lilly & Company
- **Steven Piantadosi,** Brigham and Women's Hospital
- **Louis Fiore,** Veterans Affairs

11:00 a.m. **Break**

11:15 a.m. **Outcome Measurement Based on Real-World Data**
Moderator: **Gregory Daniel**
Panelists:
- **Elizabeth Sugar,** Johns Hopkins University
- **Sean Tunis,** Rubix Health
- **Atul Butte,** University of California at San Francisco
- **David Madigan,** Columbia University
- **Bill Crown,** OptumLabs
- **Cathy Critchlow,** Amgen

12:30 p.m. **Lunch**

1:30 p.m. **Key Considerations for Blinding in Randomized Real-World Studies**
Moderator: **Gregory Daniel**
Presenter: **Simon Skibsted,** Novo Nordisk
Panelists:

- **Rita Redberg,** University of California at San Francisco
- **Satrajit Roychoudhury,** Pfizer
- **Nancy Dreyer,** IQVIA
- **Peter Stein,** U.S. Food and Drug Administration

2:45 p.m. **Break**

3:00 p.m. **Real-World Designs and Implications for Causal Inference**
Moderator: **Gregory Daniel**
Presenter: **David Price,** University of Aberdeen
Panelists:

- **Vince Willey,** HealthCore
- **Mark Levenson,** U.S. Food and Drug Administration
- **Jesse Berlin,** Johnson & Johnson
- **Lisa LaVange,** University of North Carolina

4:15 p.m. **Open Comment Period**
Gregory Daniel

4:45 p.m. **Adjourn**

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Agenda

9:00 a.m. **Key Takeaways from Day One**
Mark McClellan, Duke-Robert J. Margolis, MD, Center for Health Policy

9:15 a.m. **Monitoring Randomized Clinical Trials that Generate RWE**

Focus Area 1: Sponsor Conduct and Monitoring Challenges
Moderator: **Mark McClellan**
Presenters:
- **Elaine Irving,** GlaxoSmithKline
- **Loretta Jacques,** GlaxoSmithKline

Panelists:
- **Adrian Hernandez,** Duke University
- **Leanne Larson,** Parexel (Association of Clinical Research Organizations)

Focus Area 2: Safety Monitoring
Moderator: **Mark McClellan**
Presenter: **Nawar Bakerly,** Salford Royal NHS Foundation Trust
Panelists:
- **Greg Ball,** Merck
- **Ellis Unger,** U.S. Food and Drug Administration

Focus Area 3: Maintaining Data Integrity
Moderator: **Mark McClellan**
Presenter: **Martin Gibson**, Salford Royal NHS Foundation Trust
Panelists:
- **Michael O'Neal,** Bioclinica (Association of Clinical Research Organizations)
- **Paul Harris,** Vanderbilt University

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

11:00 a.m. **Building a Framework for Randomized Clinical Trials: Barriers, Enablers, and Infrastructure**
Moderator: **Mark McClellan**
Panelists:
- **Lesley Curtis,** Duke University
- **Joanne Waldstreicher,** Johnson & Johnson
- **Peter Stein,** U.S. Food and Drug Administration

11:30 a.m. **Open Comment Period**
Mark McClellan

12:15 p.m. **Closing Remarks and Adjournment**
Mark McClellan

Funding for this conference was made possible in part by a cooperative agreement from the U.S. Food and Drug Administration Center for Drug Evaluation and Research. The views expressed in written conference materials or publications and by speakers and moderators do not necessarily reflect the official policies of the Department of Health and Human Services nor does mention of trade names, commercial practices, or organizations imply endorsements by the U.S. Government.