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

Second Annual Duke-Margolis Conference on Real-World Data and Evidence

National Press Club • Washington, DC
October 1, 2018

9:00 a.m. **Opening Remarks**
Gregory Daniel, Duke-Margolis Center for Health Policy

9:10 a.m. **Overview of Progress-to-Date**
Morgan Romine, Duke-Margolis Center for Health Policy

9:20 a.m. **Updates from FDA**
Jacqueline Corrigan-Curay, U.S. Food and Drug Administration

9:30 a.m. **Session I: Meeting Regulatory Standards with Fit-For-Purpose RWE**
Moderator: Gregory Daniel

Part of any regulatory decision is the consistent application of a statutorily defined substantial evidence standard. This standard affords FDA flexibility in how it weighs the sources and types of evidence within a submission against a broader totality of knowledge on the medical product, underlying disease, and treatment alternatives. This session will explore the ways in which fit-for-purpose RWE, specifically RWE derived from observational approaches, could potentially meet the established standards for adequate and well-controlled clinical investigations that underpin substantial evidence.

Highlights from Duke-Margolis Working Paper:

- *Gregory Daniel*

Reactant Panel:

- *Marc Berger, International Society for Pharmacoeconomics and Outcomes Research*
- *Nancy Dreyer, IQVIA*
- *Stacy Holdsworth, Eli Lilly and Company*
- *Richard Willke, International Society for Pharmacoeconomics and Outcomes Research*
- *Peter Stein, U.S. Food and Drug Administration*

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

11:00 a.m. **Session II: Characterizing RWD Quality and Relevancy for Regulatory Purposes**
Moderator: Gregory Daniel

This session will focus on characterizing the quality and relevancy dimensions of a fit-for-purpose real-world dataset. This includes understanding the challenges and benefits of different real-world data sources for various types of RWE development for

regulatory decision-making, and documenting the processes of cleaning, transforming and linking raw RWD to the final analyzable dataset. Discussion will be rooted in the accompanying white paper.

Highlights from Duke-Margolis White Paper:

- *Christina Silcox, Duke-Margolis Center for Health Policy*

Reactant Panel:

- *Paul Bleicher, OptumLabs*
- *Kourtney Davis, GlaxoSmithKline plc*
- *Meg Powell, Target Pharmasolutions*
- *Amy Abernethy, Flatiron Health*

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

1:15 a.m. **Session III: Overview of Ongoing and Emerging RWE Efforts**
Moderator: Gregory Daniel

This session will provide a platform for other organizations to highlight the work that they are doing to further the development and application of RWD and RWE for regulatory ends.

Presentations:

- *Jeff Allen, Friends of Cancer Research*
- *David Martin, U.S. Food and Drug Administration*
- *Cartier Esham, BIO*
- *Barbara Bierer, Multi-Regional Clinical Trials Center of Brigham and Women's Hospital and Harvard*

2:15 p.m. **Session IV: Setting Goals for 2019**
Moderator: Gregory Daniel

When we reconvene in fall 2019, what realistic progress do we want to have made? What would constitute a win for everyone working in this space? Do we have collaborative goals?

Panel Discussion:

- *Marcus Wilson, HealthCore*
- *Joe Selby, Patient-Centered Outcomes Research Institute*
- *Rich Moscicki, Pharmaceutical Research and Manufacturers of America*

2:55 p.m. **Closing Remarks**
Gregory Daniel

3:00 p.m. **Adjournment**