2023 Duke-Margolis Convening on the State of Real-World Evidence Policy

Virtual Public Meeting Agenda
September 28, 2023

12:30 pm – 4:30 pm ET

Meeting Objective: Convened by the Duke-Margolis Real-World Evidence Collaborative, this public conference will provide a venue for reviewing recent RWE Collaborative activities, strategic RWD and RWE policy developments, and promising future applications of RWD/RWE.

12:30 pm **Welcome and Overview of RWE Policy Updates**

Rachele Hendricks-Sturrup, Duke-Robert J. Margolis, MD, Center for Health Policy
M. Khair ElZarrad, U.S. Food and Drug Administration

12:50 pm **Keynote**
Donna Rivera, U.S. Food and Drug Administration

1:15 pm **Session 1: Role of Master Protocols for RWE**
Objective: Discussion will center around how research involving real-world data and real-world evidence can apply lessons learned from traditional clinical trials, which have utilized master and common protocols. Additionally, panelists will share thoughts on how different research contexts can best leverage master protocols to fit with the research question, plus data considerations for designing prospective and retrospective studies.

Moderator: Rachele Hendricks-Sturrup, Duke-Robert J. Margolis, MD, Center for Health Policy

Presentation: Overview of RWE Master Protocols White Paper
Rachele Hendricks-Sturrup, Duke-Robert J. Margolis, MD, Center for Health Policy

Panel Discussion:
Marc Berger, Independent Consultant
Elise Berliner, Cerner Enviza
Jeffrey Brown, TriNetX
Josh Fessel, National Institutes of Health

Open Discussion and Q&A

2:15 pm **Break**

2:25 pm **Session 2: Evaluating Real-World Efficacy in Patient Subgroups**
Objective: There is measurable value in understanding the individual and/or combined influence of clinical and demographic characteristics among patient subgroups, especially in cases where such characteristics can be a proxy for observable variations in treatment effects. In this session, we describe opportunities and challenges to leveraging RWD to estimate and measure treatment effects among and across patient subgroups. We will

also discuss important terminology considerations that accompany measurements of therapeutic effect in subgroups, supplementing clinical trials with RWD for subgroup analysis, leveraging RWD as an equitable solution to address data missingness, and leveraging RWD to improve care among patient subgroups.

Moderator: Rachele Hendricks-Sturrup, Duke-Robert J. Margolis, MD, Center for Health Policy

Presentation: Overview of Patient Subgroups White Paper
Nora Emmott, Duke-Robert J. Margolis, MD, Center for Health Policy

Panel Discussion:
Gregory Calip, Abbvie and University of Southern California
Eibhilin Hudson, Novartis
Daniela Moga, University of Kentucky

Open Discussion and Q&A

3:25 pm **Session 3: Generating Practically Relevant Evidence in the Real World**
Objective: This discussion will explore opportunities to leverage advances in real-world data collection and evaluation to integrate clinical research into the point-of-care and accelerate the adoption of learning healthcare principles. Panelists will discuss avenues for leveraging RWD at the point-of-care to improve our ability to efficiently generate evidence and improve outcomes for patients.

Moderator: Mark McClellan, Duke-Robert J. Margolis, MD, Center for Health Policy

Presentation: Overview of LHS White Paper and ACT@POC
Trevan Locke, Duke-Robert J. Margolis, MD, Center for Health Policy

Panel Discussion:
Andenet Emiru, University of California
Frederick Masoudi, Ascension
Chris Lindsell, Duke Clinical Research Institute
Sally Okun, Clinical Trials Transformation Initiative
Hilary Marston, U.S. Food and Drug Administration

4:25 pm **Closing Remarks and Adjournment**
Mark McClellan, Duke-Robert J. Margolis, MD, Center for Health Policy

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