Duke | MARGOLIS CENTER *for* Health Policy

Developing Real-World Data and Evidence to Support Regulatory Decision-Making

National Press Club • Washington, DC
October 3, 2019

Agenda

The U.S. Food and Drug Administration's (FDA) Real-World Evidence (RWE) Framework sets forth three main considerations for evaluating RWE for regulatory use: (1) the real-world data (RWD) are fit-for-use; (2) the trial or study design used to generate RWE can provide adequate scientific evidence to answer or help answer the regulatory question; and (3) the study conduct meets FDA regulatory requirements. In this conference, stakeholders will focus on assessment of whether RWD are fit-for use, as well as discuss insights into methodological approaches and potential uses of real-world endpoints that could enhance the ability to draw causal inferences in real-world studies.

9:00 a.m. **Welcome and Overview**

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

9:05 a.m. **Welcome and Update from FDA**

- **Jacqueline Corrigan-Curay,** U.S. Food and Drug Administration

9:15 a.m. **Emerging Insights into the Development of RWE from Randomized Designs**

- **Adrian Hernandez,** Duke University
- **Peter Stein,** U.S. Food and Drug Administration

9:35 a.m. **Session I: Establishing a High-Quality RWD Ecosystem**

Objective: In this session, panelists will discuss considerations on how to develop and foster a RWD ecosystem that facilitates the collection and generation of high-quality RWD from diverse sources for use in research, including enhancing standardization, protecting patient privacy, and increasing interoperability.

Moderator: **Mark McClellan**
Panel Discussants:

- **Adam Asare,** University of California, San Francisco
- **Wendy Rubinstein,** American Society of Clinical Oncology CancerLinQ
- **Nancy Yu**, RDMD
- **Kevin Haynes,** HealthCore

10:35 a.m. **Break**

10:50 a.m. **Session II: Curating and Assessing Fit-for-Use RWD Derived from Electronic Health Records**

Objective: This session will focus on evaluating whether RWD are fit-for-use. This evaluation requires addressing fundamental questions related to the reliability of RWD as data undergo transformations from inputs provided by a source, to raw data outputs, and are ultimately curated into an analytic dataset for analysis. Panelists will explore

considerations for curation of RWD from electronic health records (EHRs) and opportunities to assess and improve data reliability.

Moderator: **Mark McClellan**
Presenter: **Keith Marsolo,** Duke University
Panel Discussants:

- **Jeffrey Brown,** Harvard Pilgrim Health Care Institute
- **Kristijan Kahler,** Novartis
- **Shaun Grannis,** Regenstrief Institute
- **Robert Ball,** U.S. Food and Drug Administration

12:00 p.m. **Lunch**

12:45 p.m. **Session III: Leveraging Digital Technology for Patient-Generated Health Data**
Objective: Patient-Generated Health Data (PGHD) is increasingly available as real-world data collected through websites, mobile health applications, and wearables and other connected devices and can provide routinely collected, high-frequency, longitudinal data on large numbers of patients to inform clinical studies. In this session, presenters will discuss collecting and utilizing PGHD and panelists will explore considerations for evaluating PGHD reliability.

Moderator: **Mark McClellan**
Presenters:

- **Ernesto Ramirez**, Evidation
- **Angela Dobes,** Crohn's & Colitis Foundation

Panel Discussants:

- **John Reites**, THREAD
- **Jacqueline Law**, Roche
- **Elizabeth Kunkoski,** U.S. Food and Drug Administration

1:45 p.m. **Session IV: Methodological and Analytical Considerations for Observational Studies**
Objective: The RWE framework considers whether the study design is adequate to answer the study question. There is interest from stakeholders on whether and how observational designs can inform questions around product effectiveness. Panelists will explore observational study design and offer additional insights into methodological approaches that might enhance our ability to draw causal inferences from non-randomized designs.

Moderator: **Mark McClellan**
Presenters:

- **Til Stürmer,** University of North Carolina
- **William Crown,** OptumLabs
- **Sebastian Schneeweiss**, Harvard University

Panel Discussants:

- **Lucinda Orsini,** ISPOR
- **Patrick Ryan,** Janssen (OHDSI)
- **Kristin Sheffield,** Eli Lilly & Co.
- **David Martin,** U.S. Food and Drug Administration

2:55 p.m. **Break**

3:05 p.m. **Session V: Opportunities to Ascertain Endpoints in Routine Clinical Care Settings**
Objective: This session will explore opportunities for leveraging endpoints used in real-world studies. Presenters will describe ongoing work in oncology to identify, collect, and validate real-world endpoints, and panelists will also discuss the applicability of regulatory requirements to these endpoints.

Moderator: **Mark McClellan**
Presenter: **Jeffrey Allen,** Friends of Cancer Research
Panel Discussants:

- **Sean Khozin,** U.S. Food and Drug Administration
- **Nicole Mahoney,** Flatiron Health
- **Andrew Norden,** COTA
- **Jonathan Hirsch,** Syapse

4:05 p.m. **Open Comment Period**

4:15 p.m. **Closing Remarks and Adjournment**