The National Academies of | SCIENCES ENGINEERING MEDICINE

Forum on DRUG DISCOVERY, DEVELOPMENT, and TRANSLATION

Examining the Impact of Real-World Evidence on Medical Product Development: A Three-Part Workshop Series

Workshop Two: Practical Approaches

March 6–7, 2018

National Academy of Sciences Building, Room 120
2101 Constitution Ave. NW, Washington, DC 20418

The National Academies of Sciences, Engineering, and Medicine (National Academies) is convening a three-part workshop series examining how real-world evidence development and uptake can enhance medical product development and evaluation. The workshops will advance discussions and common knowledge about complex issues relating to the generation and utilization of real-world evidence, including fostering development and implementation of the science and technology of real-world evidence generation and utilization.

- Workshop One (*September 19-20, 2017*) focused on how to align incentives to support collection and use of real-world evidence in health product review, payment, and delivery. Incentives need to address barriers impeding the uptake of real-world evidence, including barriers to transparency.
- **Workshop Two (*March 6-7, 2018*) will illuminate what types of data are appropriate for what specific purposes and suggest practical approaches for data collection and evidence use by developing and working through example use cases.**
- Workshop Three (*July 17-18, 2018*) will examine and suggest approaches for operationalizing the collection and use of real-world evidence.

DAY 1: March 6, 2018

8:30 a.m. Breakfast Available Outside the Room 120

8:40 a.m. **Welcome and Opening Remarks**

GREG SIMON, *Workshop Series Co-Chair*
Investigator
Kaiser Permanente Washington Health Research Institute

MARK MCCLELLAN, *Workshop Series Co-Chair*
Director
Duke-Margolis Center for Health Policy

SESSION I: WHEN CAN WE TRUST REAL-WORLD DATA?

Session discussion questions:

- Can we accept more data variance when there is a lower risk associated with the treatment?
- Are there different tiers of data quality depending on the intended use of the evidence?
- Can we identify an overarching effects on data collection? In other words, is there some predictor of systemic error rather than random error?

9:00 a.m. **Introduction to the use case and presentation of background materials**
(*Presented by the workgroup participants for this session*)

9:20 a.m. **Open discussion of session discussion questions with audience**

10:40 a.m. **BREAK**
(*Workgroup participants gather to synthesize audience feedback on principles*)

11:00 a.m. **Workgroup presents synthesis of audience feedback**

SESSION II: WHEN CAN WE TRUST REAL-WORLD TREATMENT?

Session discussion questions:

- When does variation in treatment fidelity or adherence yield a valid signal about real-world effectiveness, and when is it just noise?
- When can we trust real-world clinicians to adequately monitor participant safety and respond appropriately to adverse events?

11:20 a.m. **Introduction to the use case and presentation of background materials**
(*Presented by the workgroup participants for this session*)

11:40 p.m. **Open discussion of session discussion questions with audience**

1:00 p.m. **BREAK** (Lunch available Outside Room 120)
(*Workgroup participants gather to synthesize audience feedback on principles*)

2:00 p.m. **Workgroup presents synthesis of audience feedback**

SESSION III: WHEN CAN WE LEARN FROM REAL-WORLD TREATMENT ASSIGNMENT?

Session discussion questions:

- When can we trust inference from cluster-randomized or stepped-wedge study designs?
- Under what conditions can we trust inference rom observational or naturalistic comparisons?
- How could we judge the validity of observational comparisons in advance, rather than waiting until we've observed the result?

2:20 p.m. **Introduction to the use case and presentation of background materials**
(*Presented by the workgroup participants for this session*)

2:40 p.m. **Open discussion of session discussion questions with audience**

4:20 p.m. **BREAK**
(*Workgroup participants gather to synthesize audience feedback principles*)

4:40 p.m. **Workgroup presents synthesis of audience feedback**

5:00 p.m. **ADJOURN WORKSHOP DAY 1**

DAY 2: MARCH 7, 2018

8:30 a.m. Breakfast Available Outside the Room 120

SESSION IV: SYNTHESIZING THE USE CASES

Session Objective:

- Discuss synthesized feedback from each session on Day 1
- Consider components of a "checklist" of considerations for using real-world evidence

9:00 a.m. **Discussion of synthesized output from each session on Day 1 with audience**

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10:40 a.m. **BREAK**

11:00 a.m. **Discussion of potential components to a "checklist" for using RWE with audience**

12:30 p.m. **ADJOURN WORKSHOP DAY 2**

Future Workshop Objectives

WORKSHOP THREE. Examine and suggest approaches for operationalizing the collection and use of real-world evidence. *(July 17-18, 2018, Washington, DC)*

- Applications for using real-world evidence to supplement traditional clinical trials, pragmatic/effectiveness trials, or routine clinical application.
- Mechanisms for determining which discrete types of real-world evidence could support regulatory decisions.
- Operational challenges and barriers for generating and incorporating real-world evidence in the context of a learning health system and how clinicians can best be involved in the collection and utilization of real-world evidence.