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 One: Incentives

September 19–20, 2017

National Academy of Sciences Building, Lecture Room
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.

This first workshop will include discussions, and background materials, that address:

- **Aligning incentives and addressing barriers to support collection and use of real-world evidence in health product review, payment, and delivery.**

Workshops TWO and THREE will foster discussions that will

- Illuminate what types of data are appropriate for what specific purposes and suggest approaches for data collection that match the right data to the right questions. (Q1 2018)
- Examine and suggest approaches for operationalizing the collection and use of real-world evidence. (Q3 2018)

DAY 1: SEPTEMBER 19, 2017

8:00 a.m. Breakfast Available Outside the Lecture Room

KEYNOTE ADDRESS

8:30 a.m. **Vision and Goals of a Collaborative, Practical, and Sustainable Real-World Evidence Program**

SCOTT GOTTLIEB
Commissioner
U.S. Food and Drug Administration

8:50 a.m. **Discussion with Audience**

SESSION I SEEING OUR DESTINATION

Session Objectives:

- Explore what relevant facts the ultimate end-users of evidence need to know in order to make informed decisions about using medical products.
- Discuss possible approaches to generating such fit-for-purpose evidence.

Moderator: Andy Bindman, University of California, San Francisco

9:00 a.m. **Payer perspective**

MICHAEL SHERMAN
Senior VP and Chief Medical Officer
Harvard Pilgrim Health Care

9:20 a.m. **Delivery System perspective: Integrated Care Model at Kaiser**

MICHAEL HORBERG
Executive Director, Research Community Benefit, and Medicaid Strategy
Executive Director, Mid-Atlantic Permanente Research Institute
Kaiser Permanente

9:40 a.m. **Delivery System perspective: Academic health system**

DANIEL FORD
Director, Institute for Clinical and Translational Research
Johns Hopkins School of Medicine

10:00 a.m. **Patient-focused perspective**

SHARON TERRY
President and Chief Executive Officer
Genetic Alliance

10:20 a.m. **Discussion with Audience**

Additional invited discussants:

JOANNE WALDSTREICHER
Chief Medical Officer
Johnson&Johnson

ELEANOR PERFETTO (*invited*)
Senior Vice President, Strategic Initiatives
National Health Council

11:10 a.m. **BREAK**

11:30 a.m. **Key Messages and Themes from the September 13th FDA/Duke-Margolis Workshop: Generating Fit-for Purpose Evidence**

MARK MCCLELLAN
Director
Duke-Margolis Center for Health Policy

11:50 a.m. **Discussion with Audience**

12:00 pm **BREAK** (Lunch available Outside the Lecture Room)

SESSION II LEARNING FROM SUCCESS

Session Objectives:

- Highlight successful completed and ongoing initiatives that could potentially be examined for real-world evidence collection and use.
- Explore the features that led to the success in the given examples and how they could apply to future applications:
    - Conditions likely to make innovation successful; and
    - Potential ways to recreate those conditions to make real-world evidence use more routine.

Moderator: Greg Simon, Kaiser Permanente Washington Health Research Institute

1:00 p.m. **Generalizing and Scaling the Salford Lung Studies**

MARTIN GIBSON
Chief Executive Officer
Northwest EHealth

MARIE KANE
Chief Operating Officer
Northwest EHealth

1:30 p.m. **Using Sentinel to Evaluate Effectiveness or Efficacy**

RICH PLATT
Professor and Chair, Dept. of Population Medicine
Harvard Medical School

1:50 p.m. **Applying Lessons learned from Device Registries to Other Treatment Types**

RACHAEL FLEURENCE
Executive Director
NEST Coordinating Center

2:10 p.m. **Discussion with Audience**

Additional invited discussants:

JOHN GRAHAM
Head, Value Evidence and Outcomes
GlaxoSmithKline

RACHEL SHERMAN
Deputy Commissioner
U.S. Food and Drug Administration

3:00 p.m. **BREAK**

SESSION III GETTING UNSTUCK: ALIGNING INCENTIVES

Session Objectives:

In a series of presentations, discuss with treatment developers and evidence generators:

- Incentives maintaining the current data generation process; and
- Disincentives and potential barriers to incorporation of real-world evidence.

Moderator: Petra Kaufmann, National Center for Advancing Translational Sciences, NIH (*invited*)

3:20 p.m. **Contract Research Organization Perspective**

JOHN DOYLE (*invited*)
Senior Vice President and Managing Director
QuintilesIMS

3:40 p.m. **Product Developer Perspective**

ELLIOTT LEVY
Senior Vice President, Global Devlopment
Amgen

BRIAN D. BRADBURY
Executive Director, Center for Observational Research
Amgen

4:00 p.m. **Academic Researcher Perspective**

DANIEL FORD
Director, Institute for Clinical and Translational Research
Johns Hopkins School of Medicine

4:20 p.m. **Data Stewards: Organizations with Large Data Sources**

MARCUS WILSON
President
HealthCore, Inc.

4:40 p.m. **Discussion with Audience**

Additional invited discussants:

MICHAEL HORBERG
Executive Director, Research Community Benefit, and Medicaid Strategy
Executive Director, Mid-Atlantic Permanente Research Institute
Kaiser Permanente

ANNA MCCOLLISTER-SLIPP
Chief Advocate for Participatory Research, Scripps Translational Science Institute
Co-founder, Galileo Analytics

5:30 p.m. **ADJOURN WORKSHOP DAY 1**

DAY 2: SEPTEMBER 20, 2017

8:00 a.m. Breakfast Available Outside the Lecture Room

8:30 a.m. **Recap Day One and Discussion with Workshop Participants**

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

KEYNOTE ADDRESS

9:00 a.m. **Keynote: False Precision and Estimating the Reliability of Effects with the Traditional Evidence Generating Process**

ROB CALIFF
Vice Chancellor, Health Data Science, Duke University
Verily Life Sciences

SESSION IV GETTING UNSTUCK: MYTH-BUSTING

Session Objective: Examine ideas—and misconceptions—about the necessity and acceptability of established evidence-generation practices.

Moderator: Rob Califf, Duke University and Verily Life Sciences

9:30 a.m. **Moving from "One Study at a Time" to "All by All" Analyses**

PATRICK RYAN
Senior Director and Head, Epidemiology Analytics
Janssen Research and Development

9:50 a.m. **Regulatory Perspective**

JANET WOODCOCK
Director
Center for Drug Evaluation and Research
U.S. Food and Drug Administration

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

10:30 a.m. **Academic Researcher Perspective**

RORY COLLINS
Head of Nuffield Dept of Population Health
University of Oxford

10:50 a.m. **Medical Product Developer Perspective**

JOHN GRAHAM
Head, Value Evidence and Outcomes
GlaxoSmithKline

11:10 a.m. **Discussion with Audience**

Additional invited discussant:

DEVEN MCGRAW
Deputy Director, Health Information Privacy
Office for Civil Rights
U.S. Department of Health and Human Services

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

Future Workshop Objectives

WORKSHOP TWO. Illuminate what types of data are appropriate for what specific purposes and suggest approaches for data collection that match the right data to the right questions. *(Q1 2018)*

- Precise language and nomenclature for describing data, data collection activities, and data sources.
- Sources of data that are curated, standardized, and analyzed to derive real-world evidence, such as safety surveillance, observational studies, registries, claims, or patient-centered outcomes research.
- Gaps in data collection activities, and priority areas and pilot opportunities that real-world evidence incorporation could address.
- Standards and methodologies for collecting and analyzing real-world evidence in support of new indications or postapproval studies, and the circumstances under which that evidence could be applied.

WORKSHOP THREE. Examine and suggest approaches for operationalizing the collection and use of real-world evidence. *(Q3 2018)*

- 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.