The National Academies of
SCIENCES • ENGINEERING • MEDICINE

Forum on
DRUG DISCOVERY, DEVELOPMENT, and TRANSLATION

Real-World Evidence Generation and Evaluation of Therapeutics: A Workshop

October 19, 2016

National Academy of Sciences Building, Room 120
2101 Constitution Ave, N.W., Washington, D.C. 20418

BACKGROUND AND WORKSHOP OBJECTIVES

The traditional process for evaluating new therapeutics does not produce the evidence that patients, clinicians, and payers need for real-world decisions. The volume and complexity of information about individual patients is greatly increasing with use of electronic records and personal devices. Possibilities for medical product development in the context of this wealth of real-world data are great, ranging from the ability to determine both large-scale and patient-specific effects of treatments to assessing how therapeutics affect patients' lives through measurement of lifestyle changes. However, mechanisms to facilitate efficient use of real-world data to meet the decision-making needs of myriad stakeholders have not been established. An ad hoc committee will plan and conduct a one-day public workshop that will examine opportunities and challenges for incorporating real-world evidence into evaluation of medical products.

Subject matter experts will be invited to participate in the workshop through presentations and discussions that will consider:

- Quality of data from real-world sources, including:
  - Relevance and validity of different sources of real-world data (e.g., user-collected, practice-based) in the context of different clinical/scientific questions; and
  - Strengths and limitations of different data sources at different stages of treatment development and licensing process.
- Methodologies and best practices for high-quality real-world evidence generation and application, including:
  - Innovations in clinical trial design to maximize value of information for the full range of stakeholders;
  - Considerations of how evidence generation from existing studies could potentially inform the design of future clinical trials and amplify understanding of product efficacy;
  - Discussion of how shared goals of payers and regulators can better align evidence generation processes used for regulatory evaluation and decisions on use by payers; and.
  - Re-evaluation of traditional distinctions between goals and methods of pre-approval and post-approval research.
- Other novel methodologies and approaches to improve development and evaluation of products using real-world evidence, including:
  - Use of web-based or digital technologies to enhance clinical trial evidence collection and participation.
  - Techniques and case-studies for effectively using electronic health record (EHR) data.

8:30 A.M. SESSION I: BREAKING THE MOLD – STAKEHOLDER PRIORITIES FOR IMPROVING EVIDENCE GENERATION

Session Objectives

- Examine different stakeholder evidence needs to support decision making and identify shared goals
- Discuss priorities for facilitating use of real-world evidence to address stakeholder needs
- Discuss aligning incentives to maximize generation and sharing of useful evidence

8:30 a.m. **Opening Remarks and Introductions**

STEVEN GALSON, *Workshop Co-Chair*
Senior Vice President for Global Regulatory Affairs and Safety
Amgen Inc.

GREG SIMON, *Workshop Co-Chair*
Investigator, Group Health Research Institute
Chair, Scientific Advisory Board, Depression and Bipolar Support Alliance

8:45 a.m. **Keynote**

ROBERT CALIFF
Commissioner
U.S. Food and Drug Administration

9:05 a.m. **Stakeholder Perspectives**

JOHN CARROLL
Professor of Medicine
Co-Medical Director, Cardiac and Vascular Center
University of Colorado Hospital

JOSEPH CHIN
Deputy Director, Coverage and Analysis Group
Centers for Medicare and Medicaid Services

ZVI FRANKEL
Patient and Consumer Advocate

RHONDA ROBINSON BEALE
Chief Medical Officer
Blue Cross of Idaho

RACHEL SHERMAN
Deputy Commissioner for Medical Products and Tobacco
U.S. Food and Drug Administration

PATRICK VALLANCE
President, Pharmaceuticals Research and Development
GlaxoSmithKline

9:30 a.m. **Moderated Discussion with Session I Speakers and Audience**

ROBERT CALIFF, *Moderator*
Commissioner
U.S. Food and Drug Administration

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

11:00 A.M. SESSION II: WHAT CAN WE LEARN FROM REAL-WORLD DATA?

Session Objectives

- Examine different sources of real-world data (e.g., user-collected, practice-based) and consider their reliability in the context of different clinical/scientific questions
- Discuss strengths and limitations of different data sources at different stages of treatment development and licensing process.

11:00 a.m. **Background and Session Objectives**

JOHN HERNANDEZ, *Moderator*
Head of Health Economics, Value and Access
Verily Life Sciences

NIGAM SHAH, *Moderator*
Associate Professor of Medicine, Biomedical Information Research
Stanford University

11:05 a.m. **Sources for and Practical Use of Real-World Data**

Data Sharing and Linking Records Across EHR Vendors

JON WHITE
Deputy National Coordinator for Health Information Technology
Office of the National Coordinator for Health Information Technology

PCORnet and Clinical Data Research Networks

RUSSELL ROTHMAN
Director, Center for Health Services Research
Chief, Internal Medicine and Pediatrics
Vanderbilt University

Potential for Data Analytics

DAVID DORE
Vice President, Epidemiology & Principal Epidemiologist
Optum Life Sciences

Using Data from Activities on Mobile Devices

LUCA FOSCHINI
Cofounder and Chief Data Scientist
Evidation Health

11:45 a.m. **Moderated Discussion with Session II Speakers and Audience**

12:30 p.m. **Lunch**

1:15 P.M. SESSION III: THE PROMISE OF REAL-WORLD EVIDENCE – STRATEGIES FOR BUILDING FROM SUCCESSFUL USE CASES

Session Objectives

- Discuss examples of successful approaches to generating and incorporating real-world evidence into development and evaluation of medical products.
- Identify opportunities and challenges to scaling up successful practices and adapting them to new purposes.

1:15 p.m. **Background and Session Objectives**

JESSE BERLIN, *Moderator*
Vice President and Global Head of Epidemiology
Johnson & Johnson

CATHY CRITCHLOW, *Moderator*
Vice President and Head, Center for Observational Research
Amgen, Inc

1:20 p.m. **Successful Use Cases of Real-World Evidence**

Case Study #1: Salford Lung Study

ANDREW RODDAM
Vice President and Head of Real-World Evidence
GlaxoSmithKline

Case Study #2: Transcatheter Valve Therapy (TVT) Registry

MICHAEL MACK
Medical Director of Cardiothoracic Surgery
Baylor Health Care System

Case Study #3: Sentinel Initiative

LESLEY CURTIS
Professor of Medicine
Director for Pragmatic Health Services Research
Duke Clinical Research Institute

Case Study #4: Observational Health Data Sciences and Informatics (OHDSI)

NIGAM SHAH
Associate Professor of Medicine, Biomedical Information Research
Stanford University

2:20 p.m. **Moderated Discussion with Stakeholder Reaction Panel**

LAURA DEMBER
Professor of Medicine
Renal, Electrolyte, and Hypertension Division
University of Pennsylvania

LOUIS FIORE
Executive Director
Massachusetts Veterans Epidemiology Research and Information Center

MARC BERGER
Vice President, Real World Data and Analytics
Pfizer Inc.

RHONDA ROBINSON BEALE
Chief Medical Officer
Blue Cross of Idaho

SEAN TUNIS
Founder, President, and Chief Executive Officer
Center for Medical Technology Policy

3:30 p.m. **Break**

3:45 P.M. SESSION IV: REAL-WORLD EVIDENCE OF THE FUTURE – POTENTIAL STRATEGIES FOR A WAY FORWARD

Session Objectives

- Outline an ideal future state for incorporating real-world evidence into evaluation of medical products.
- Identify short-term and long-term next steps at any stage of clinical research to achieve seamless use of real-world evidence.
- Discuss incentives that should be explored.

3:45 p.m. **Reflecting on Tactics and Strategies for a Way Forward: Discussion with Workshop Co-chairs, Session Moderators, Panelists, and Audience**

MARK MCCLELLAN, *Moderator*
Director
Margolis Center for Health Policy
Duke University

STEVEN GALSON, *Workshop Co-Chair*
Senior Vice President for Global Regulatory Affairs and Safety
Amgen Inc.

GREG SIMON, *Workshop Co-Chair*
Investigator, Group Health Research Institute
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Verily Life Sciences

FREDA LEWIS-HALL
Chief Medical Officer and Executive Vice President
Pfizer

NIGAM SHAH
Associate Professor of Medicine, Biomedical Information Research
Stanford University

RACHEL SHERMAN
Deputy Commissioner for Medical Products and Tobacco
U.S. Food and Drug Administration

4:20 p.m. **Moderated Discussion with Session IV Panel and Audience**

5:00 p.m. **Adjourn**