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.
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
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**

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**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  
Chair, Scientific Advisory Board, Depression and Bipolar Support Alliance
JESSE BERLIN  
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Patient and Consumer Advocate

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Head of Health Economics, Value and Access  
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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