Center for Health Policy at BROOKINGS

Facilitating Biomarker Development: Strategies for Scientific Communication, Pathway Prioritization, Data-Sharing, and Stakeholder Collaboration

Embassy Suites - Convention Center • Washington, DC
October 27, 2015

Objectives: The objectives for this expert workshop are to: 1) discuss the common lexicon developed by FDA and NIH for the field of biomarker development; 2) using case studies, explore the biomarker characteristics (including its Context of Use) that can be used to determine whether and under what circumstances a biomarker should be targeted for qualification, and 3) develop an initial set of strategies that can help to ensure better cross-sector collaboration and communication in the area of biomarker development and qualification.

8:30 a.m. **Registration**

9:00 a.m. **Welcome, Overview, and Meeting Objectives**
Greg Daniel, Managing Director, Evidence Development & Innovation, Center for Health Policy; Fellow, Economic Studies, The Brookings Institution

9:15 a.m. **Biomarker Development and Qualification: Framing the Major Issues**
Objective: Provide an overview of the major incentives and barriers to biomarker development and qualification from the regulatory, scientific, and industry perspective as well as an overview of how this event fits in with the agency's broader efforts to improve biomarker development and regulatory use.

Presentation: Robert Califf, Deputy Commissioner for Medical Products and Tobacco, U.S Food and Drug Administration

9:30 a.m. **Session I: Developing a Standard Glossary of Terms in Biomarker Development**
Objective: Varied specification and irregular use of biomarker-related terminology have hindered progress in the field of biomarker development. In order to achieve greater clarity and facilitate more effective collaboration, FDA and NIH have partnered to develop a common lexicon. This lexicon will be circulated in advance to attendees, and a representative of the joint working group will present on the history and purpose of their development as well as a select few of the terms that are most relevant to the day's discussion. This session will include a brief discussion of the definitions and will focus on targeted questions related to the dissemination and acceptance of those definitions by the broader community. Participants will be able to submit written feedback on those definitions after the meeting.

Presentation: Lisa McShane, Mathematical Statistician, National Cancer Institute

10:15 a.m. **Break**

10:30 a.m. **Session II: Qualification or Individual Drug Development Program? Determining the Appropriate Pathway for Biomarker Development and Regulatory Acceptance**

Objective: Uncertainty about when, why, and how a biomarker should be developed to support regulatory acceptance often contributes to confusion. This session will begin with a presentation from FDA on the three pathways for biomarker development. The session will then use case studies to highlight essential elements that affect the feasibility and likely value of a given biomarker development effort including the Context of Use and the quality and availability of data. The first case study is TKV as a prognostic marker polycystic kidney disease, which was recently qualified by FDA; the second case study is EGFR status as a predictive marker for EGFR-targeted therapy in NSCLC, which has been used in several individual drug development programs.

Lead presentation: **Defining the two pathways for biomarker development**

- Christopher Leptak, Biomarker and Companion Diagnostics Lead, Center for Drug Evaluation and Research, U.S. Food and Drug Administration

Case Study I: Total Kidney Volume as a prognostic marker for Polycystic Kidney Disease

- Lead presenter: Aliza Thompson, Medical Officer, Division of Cardiovascular and Renal Products, Center for Drug Evaluation and Research, U.S. Food and Drug Administration

Case Study II: **EGFR status as a predictive marker for EGFR-targeted therapy in lung cancer**

- Lead presenter: Sean Khozin, Senior Medical Officer, Office of Hematology and Oncology Products, Center for Drug Evaluation and Research, U.S. Food and Drug Administration

Panelists:

- Ronald Perrone, Associate Chief, Division of Nephrology, Tufts Medical Center, Professor of Medicine, Tufts University School of Medicine
- Steve Broadbent, Chief Operating Officer, Critical Path Institute
- Gary Kelloff, Special Advisor, National Cancer Institute
- Frank Sistare, Associate Vice President, Investigative Laboratory Sciences, Safety Assessment and Laboratory Animal Resources, Merck
- John Walsh, President, COPD Foundation

12:15 p.m. **Lunch**

1:15 p.m. **Session III: Strategies for Improving Data Standardization and Sharing**

Objective: One of the key challenges in the field of biomarker development is the issue of data sharing (e.g., due to disaggregated data, differing data standards, and proprietary concerns). This session will provide an opportunity to: 1) discuss the main barriers to biomarker data sharing, including issues related to standardization, aggregation, and dissemination, and 2) identify possible strategies to address those barriers.

Panelists:

- Gabriela Lavezzari, Assistant Vice President, Scientific Affairs, Pharmaceutical Research and Manufacturers of America
- David Wholley, Director, Biomarkers Consortium
- Paul Watkins, Director, The Hamner-UNC Institute for Drug Safety Sciences, Professor of Medicine, Professor of Toxicology, and Professor of Experimental Therapeutics, UNC
- Rebecca Kush, President and Chief Executive Officer, Clinical Data Interchange Standards Consortium

2:30 p.m. **Break**

2:45 p.m. **Session IV: Facilitating Collaboration and Cross-Sector Communication**

Objective: There are a number of organizations, partnerships, and consortia that are working to develop biomarkers in specific areas. However, their efforts are not well-coordinated, and there is a need to identify approaches to collaboration and communication that can: 1) help to identify and prioritize areas of highest unmet need in the field, and 2) ensure that consortia and other key stakeholders are working collaboratively and sharing critical information. This session will serve to identify and explore possible approaches to achieving these goals in the short and long-term.

Opening remarks:

- Mark McClellan, Senior Fellow and Director, Health Care Innovation and Value Initiative, The Brookings Institution

Panelists:

- Janet Woodcock, Director, Center for Drug Evaluation and Research, U.S. Food and Drug Administration
- Pamela McInnes, Deputy Director, National Center for Advancing Translational Science, National Institutes of Health
- Martha Brumfield, President and Chief Executive Officer, Critical Path Institute
- David Wholley, Director, Biomarkers Consortium
- Nathalie Seigneuret, Senior Scientific Project Manager, Innovative Medicines Institute
- Jeff Allen, Executive Director, Friends of Cancer Research

3:45 p.m. **Closing remarks**

4:00 p.m. **Adjournment**

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