Advancing the Discipline of Regulatory Science for Medical Product Development: An Update on Progress and a Forward-Looking Agenda
An IOM Workshop

October 20–21, 2015
Keck Center
500 5th Street, NW Room 100
Washington, DC 20001

Background and Workshop Objectives:
The Food and Drug Administration (FDA) defines regulatory science as the science of developing new tools, standards, and approaches to assess the safety, effectiveness, quality, toxicity, public health impact, or performance of FDA regulated products. Since its inception, the IOM’s Forum on Drug Discovery, Development, and Translation has focused on the need for strengthening the scientific basis of drug regulation. In February 2010, the Forum held a workshop, Building a National Framework for the Establishment of Regulatory Science for Drug Development, that examined the state of the science of drug regulation and considered approaches to enhance regulatory science. In September 2011, the Forum held another workshop, Strengthening a Workforce for Innovative Regulatory Science in Therapeutics Development, that considered opportunities and needs for advancing innovative regulatory science through workforce and career development. Over the past several years, models to support the discipline have advanced. FDA’s Centers of Excellence in Regulatory Science and Innovation enhance training and educational opportunities for regulatory scientists. Private funders have also established programs: for example, in 2011 the Burroughs Wellcome Fund launched Innovations in Regulatory Science Awards (IRSA), which aim to strengthen regulatory systems capacity by funding regulatory science-based research and collaborations.

This workshop will provide a venue to review progress in building the foundations of regulatory science and to explore a forward-looking agenda for bolstering the field. Participants will examine the current state and scope of the discipline, highlight opportunities to address barriers to success, and explore ways to foster collaboration. The workshop objectives are to:

- Explore current regulatory science priorities and strategies in federal, academic, and private sector settings.
- Consider the current state of regulatory science as a discipline.
  - Discuss professional training successes.
  - Highlight opportunities to further support training, workforce, and career development.
- Explore the core components of a robust discipline of innovative regulatory science.
  - Consider gaps and key opportunities to address needs to support the discipline of innovative regulatory science.
- Examine needs and barriers to collaboration among, across, and within the public and private sectors.
Day One

8:00 a.m.  Breakfast Available

8:30 a.m.  Opening Remarks

MARTIN PHILBERT, *Workshop Co-Chair*
Professor and Dean
University of Michigan School of Public Health

ALASTAIR WOOD, *Workshop Co-Chair*
Partner, Symphony Capital
Professor of Medicine and Pharmacology, Weill Cornell School of Medicine

SESSION I: SETTING THE STAGE FOR INNOVATION IN REGULATORY SCIENCE

**Session Objectives:**
- Introduce and discuss workshop theme.
- Highlight key scientific questions for the field of innovative regulatory science, focusing on the role of information as it is generated across regulatory science domains and ways that it can be better put to use.
- Discuss how new capabilities and access to new information could advance regulatory science for medical product development.
- Highlight operational challenges.

8:40 a.m.  Background and Session Objectives

*Session Chair:* Alastair Wood, Partner, Symphony Capital, Professor of Medicine and Pharmacology, Weill Cornell School of Medicine (*Workshop Co-Chair*)

8:45 a.m.  Workshop Theme and Framework:

**Innovation in Regulatory Science Through Integration of Information**

*Transformation of Our Ability to Generate, Analyze, Integrate and Share Information Across Regulatory Science Applications*  

RUSS ALTMAN  
The Kenneth Fong Professor of Bioengineering, Genetics, Medicine & (by courtesy) Computer Science  
Stanford University
9:00 a.m.  
*Role of New Sources of Information and Information Sciences in Regulatory Science*

JIM STEVENS  
Distinguished Research Fellow  
Eli Lilly

9:15 a.m.  
*Value of Information to Inform Decision Making Under Uncertainty*

KATHERINE VON STACKELBERG  
Research Scientist  
Harvard Center for Risk Analysis

9:30 a.m.  
*Fusing Randomization With EHR ‘Big Data’ For Smarter Evidence Generation On Approved Medical Products*

DEREK ANGUS  
Distinguished Professor and Mitchell P. Fink Endowed Chair, Department of Critical Care Medicine  
University of Pittsburgh

9:45 a.m.  
**Panel Discussion and Audience Q&A (30 mins)**

10:15 a.m.  
**BREAK (15 mins)**

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**SESSION II: LEARNING LESSONS THROUGH CONSIDERATION OF REGULATORY SCIENCE APPLICATIONS**

*Session Objectives:*

- Discuss how enhanced approaches to obtaining, accessing, and integrating information could advance the science throughout and across development.
- Through consideration of selected regulatory science applications, discuss current capabilities for regulatory science and strategic priorities in federal, academic, and private sectors.
- Suggest ways forward to address identified gaps and operational challenges.

10:30 a.m.  
**Background and Session Objectives**

*Session Chair: *Stephen Ostroff, Acting Commissioner, U.S. Food and Drug Administration
10:35 a.m. **Identifying and Developing Meaningful Biomarkers**

*Panel Moderator:* John Wagner, Senior Vice President, Head of Clinical and Translational Sciences, Takeda Pharmaceuticals

10:40 a.m. **Basic Science of Measurement: Metrology Principles for Biomarkers**

**Marc Salit**
Leader, Genome-Scale Measurements
National Institute of Standards and Technology (NIST)

10:50 a.m. **Opportunities to Develop Meaningful Biomarkers: Polycystic Kidney Disease Biomarker Qualification**

**Shashi Amur**
Scientific Lead, CDER’s Biomarker Qualification Program
U.S. Food and Drug Administration

11:00 a.m. **Challenges and Opportunities for Qualifying Biomarkers: An Industry Perspective**

**Gabriela Lavezzari**
Assistant VP, Science & Regulatory Advocacy
Pharmaceutical Research and Manufacturers of America (PhRMA)

11:10 a.m. **Collaborative Approaches for Developing Kidney Safety Biomarkers**

**John Michael Sauer**
Executive Director, Predictive Safety Testing Consortium (PSTC)
The Critical Path Institute

11:20 a.m. **Panel Discussion and Audience Q&A (30 mins)**

11:50 p.m. **BREAK to Lunch (60 mins)**
SESSION II, CONT’D: CONSIDERATION OF REGULATORY SCIENCE APPLICATIONS

12:50 p.m. **Clinical Trial Data Integration**

*Panel Moderator:* Rob Califf, Deputy Commissioner for Medical Products and Tobacco, U.S. Food and Drug Administration

12:55 p.m. **Developing Capabilities to Integrate and Use Data from Very Large Data Sets**

**Martin Landray**
Professor of Medicine and Epidemiology, Deputy Director Big Data Institute
University of Oxford

1:05 p.m. **Approaches to Overcoming Variance Due to Heterogeneity in Rare Disease**

**Susan Ward**
Founder and Executive Director
The TAP Collaboration

1:15 p.m. **Access To Patient Level Data From Clinical Trials**

**Perry Nisen**
Chief Executive Officer
Sanford Burnham

1:25 p.m. **The Role of Open APIs (Application Programming Interface) and the FHIR (Fast Healthcare Interoperability Resources) Platform for Enabling The Integration of Research and Clinical Care Data**

**Charles Jaffe**
Chief Executive Officer
Health Level Seven International

1:35 p.m. **Data Aggregation Across Diseases and Between Stakeholders**

**Enrique Aviles**
Director of Data Standards, Management, and Technology
The Critical Path Institute

1:45 p.m. **Panel Discussion and Audience Q&A (30 mins)**

Panelists:
- *Clinical Trial Data Integration* speakers (above), and
- Kyle J. Myers, Director, Division of Imaging, Diagnostics, and Software Reliability (DIDSR), U.S. Food and Drug Administration
2:15 p.m.  **BREAK (15 mins)**

2:30 p.m.  **Next Generation Surveillance**

*Panel Moderator:* Brian Strom, Chancellor of Rutgers Biomedical and Health Sciences, Rutgers, the State University of New Jersey

2:35 p.m.  *Integrating Systems and Capabilities to Enhance Safety Surveillance*

**RICHARD PLATT**
Professor and Chair of the Department of Population Medicine  
Harvard Pilgrim Health Care Institute

2:45 p.m.  *Harnessing Web Search Data as Complementary Signals for Pharmacovigilance*

**ERIC HORVITZ**
Distinguished Scientist & Managing Director  
Microsoft Research

2:55 p.m.  *Online Discussion Forums as Potential Sources of Adverse Drug Event Data*

**JOHN H. HOLMES**
Professor of Medical Informatics  
University of Pennsylvania

3:05 p.m.  *New Frontiers: Surveying Twitter Feeds and Other Social Media*

**JOHN BROWNSTEIN**
Associate Professor  
Harvard Medical School

3:15 p.m.  **Panel Discussion and Audience Q&A (30 mins)**

Panelists:
- *Next Generation Surveillance* speakers (above), and
- Danica Marinac-Dabic, Director, Division of Epidemiology, Center for Devices and Radiological Health (CDRH), U.S. Food and Drug Administration
3:45 p.m. **Innovation in Modeling and Integrating Information**

**Panel Moderator:** Darrell Abernethy, Associate Director for Drug Safety, Office of Clinical Pharmacology, U.S. Food and Drug Administration

3:50 p.m. *Statistical Modeling for Efficient and Adaptive Trial Designs Using Composite Endpoints*

BRIAN ALEXANDER  
Assistant Professor of Radiation Oncology  
Harvard Medical School

4:00 p.m. *Models of Clinical Trial PK/PD Translated To Population Drug Use and Exposure*

SANDY ALLERHEILIGEN  
Vice President Modeling and Simulation  
Merck

4:10 p.m. *A Quantitative and Integrative Simulation Model for Optimizing Clinical Trial Design to Measure Cognitive Changes of Alzheimer’s Disease*

BRIAN CORRIGAN  
Senior Director  
Pfizer

4:20 p.m. *Assessing the Placebo Effect and Drug Efficacy for Rare Diseases in the Brain Using fMRI*

ARIANA ANDERSON  
Assistant Research Statistician  
University of California Los Angeles

4:30 p.m. **Panel Discussion and Audience Q&A (30 mins)**

Panelists:
- *Innovation in Modeling and Integrating Information* speakers (above), and
- Klaus Romero, Director of Clinical Pharmacology, The Critical Path Institute (C-Path)

5:00 p.m. Wrap-up of Day 1

5:10 p.m. **ADJOURN**
Day Two

8:30 a.m. Welcome and Reflections from Day 1

**MARTIN PHILBERT, Workshop Co-Chair**
Professor and Dean
University of Michigan School of Public Health

**ALASTAIR WOOD, Workshop Co-Chair**
Partner, Symphony Capital
Professor of Medicine and Professor of Pharmacology, Weill Cornell School of Medicine

**SESSION III: ENVISIONING THE FUTURE OF REGULATORY SCIENCE: A FORWARD-LOOKING AGENDA**

Session Objectives:
- Discuss opportunities and priorities to advance innovative regulatory science through information.

8:35 a.m. **Disciplinary Components and Infrastructure Needs**

**Panel Moderator:** Martin Philbert, Professor and Dean, University of Michigan  
*(Workshop Co-Chair)*

8:40 a.m. **A Workforce to Bridge the Translational and Regulatory Bottlenecks in Drug Development**

**GARRET FITZGERALD**
Professor of Medicine and Pharmacology
University of Pennsylvania
8:50 a.m.  
*Core Components of Regulatory Science Curriculum*

SCOTT STEELE  
Director of Government and Academic Research Alliances  
Associate Professor of Public Health Sciences  
University of Rochester

9:00 a.m.  
*Lessons from Another Sector: Big Data at Northrop Grumman*

SAM SHEKAR  
Chief Medical Officer  
Northrop Grumman

9:10 a.m.  
*Training the Regulatory Scientist for Medical Product Development*

OWEN FIELDS  
Vice President Regulatory Strategy  
Pfizer Inc.

9:20 a.m.  
**Panel Discussion and Audience Q&A (30 mins)**

Panelists:
- *Disciplinary Components and Infrastructure Needs* speakers (above), and
- Peter Honig, Senior Vice President and Head of Worldwide Safety and Regulatory, Pfizer Inc.
- Frank Weichold, Director, Science and Innovation, Office of the Chief Scientist/Office of the Commissioner, U.S. Food and Drug Administration

9:50 a.m.  
**Day 2 Keynote (15 mins; followed by 5 mins of Q&A)**

*The Future of Regulatory Science at FDA*

STEPHEN OSTROFF  
Acting Commissioner  
U.S. Food and Drug Administration

10:10 a.m.  
**BREAK (15 mins)**

10:25 a.m.  
**Presentation of Key Themes/Suggested Paths from Session II Panel Moderators and Session Chair (4 speakers; 10 mins each)**

*Panel Introduction*

STEPHEN OSTROFF  
Acting Commissioner  
U.S. Food and Drug Administration
10:30 a.m.  *Session II moderators*

**JOHN WAGNER** (moderator of *Identifying and Developing Meaningful Biomarkers*)  
Senior Vice President, Head of Clinical and Translational Sciences  
Takeda Pharmaceuticals

**SHARON HESTERLEE** (for Rob Califf, moderator of *Clinical Trial Data Integration*)  
Chief Science Officer  
Myotonic Dystrophy Foundation

**BRIAN STROM** (moderator of *Next Generation Surveillance*)  
Chancellor of Rutgers Biomedical and Health Sciences  
Rutgers, the State University of New Jersey

**DARRELL ABERNETHY** (moderator of *Innovation in Modeling and Integrating Information*)  
Associate Director for Drug Safety, Office of Clinical Pharmacology  
U.S. Food and Drug Administration

11:10 a.m.  *Reflecting and Envisioning the Regulatory Science Discipline of 2020: Panel Discussion with Session Chairs, Panel Moderators, Panelists, and Audience*

**Panel Moderators:** Martin Philbert and Alastair Wood (*Workshop Co-Chairs*)

Panelists:
- Session II moderators (above), and
- Eileen Cannon, President, PhRMA
- Mark C. Rogers, Board Chairman, Reagan-Udall Foundation

**Discussion Questions:**
- What are the 3–5 priorities that could advance regulatory science domains?
- Do we have a cohesive approach to advancing the discipline of regulatory science? Are the strategic priorities that have been articulated and adopted by the key players aligned with, and positioned to advance innovative regulatory science?
- What investments and incentives are needed to get us there?
- How to bridge the gap from regulatory science knowledge to regulation and practice?

12:10 p.m.  **ADJOURN**