Envisioning a Transformed Clinical Trials Enterprise in the United States: Establishing an Agenda for 2020

November 7-8, 2011

20 F Street NW Conference Center
20 F Street NW
Washington, DC 20001

Background:

There is increasing recognition that the clinical trials enterprise in the United States faces substantial challenges impeding the efficient and effective conduct of clinical and translational research needed to support the development of breakthrough medicines. A gap exists between the desired state where medical care in the United States is provided solely based on high quality evidence and the reality of our limited ability to generate timely and practicable evidence. 85 percent of clinical decisions in the United States are not supported by high quality evidence. At the same time, U.S. clinical trials that generate medical evidence are becoming increasingly costly while experiencing greater setbacks. In addition, the shifting “footprint” of clinical trials toward sites outside of the United States prompts questions about the generalizability and applicability of the results of those clinical trials to the U.S. population and represents a competitive challenge for the United States.

The limited ability of the nation’s clinical trials system to support drug development and evaluation exists within a broader context of a need for a “learning health system,” where “knowledge generation is so embedded into the core of the practice of medicine that it is a natural outgrowth and product of the healthcare delivery process and leads to continual improvement in care.” An essential component of such a learning health system is a robust and well-working clinical trials enterprise to support drug development; inform quality improvement; and support surveillance, international, and comparative effectiveness research.

The IOM’s Forum on Drug Discovery, Development, and Translation has established an initiative to address challenges facing the U.S. clinical trials enterprise and to engage stakeholders in an open discussion of potentially transformative strategies to improve the efficiency and effectiveness of clinical trials.

Workshop Objectives:

- Frame the problem and discuss a vision for a clinical trials enterprise that is efficient and effective and fully integrated into the health delivery system of 2020. Define how the envisioned clinical trials enterprise differs from the current system and suggest approaches to transform our current system into a learning system.
- Consider the following core themes in framing an agenda to effect transformation of the U.S. clinical trials enterprise:
  - Providing a vision for a clinical trials enterprise in the health care system of 2020.
Developing a robust clinical trials workforce.
Aligning cultural and financial incentives.
Building an infrastructure to support a transformed clinical trials enterprise.

- Workshop presentations and panel discussions will be supported and supplemented by discussion papers prepared by participants in Forum activities. Each of the four workshop sessions will be prefaced by presentations from discussion paper authors.

November 7, 2011

8:30 a.m.  Breakfast available
9:00 a.m.  Welcome and Introductions

JEFFREY DRAZEN, Workshop Chair
Editor-in-Chief
New England Journal of Medicine

SESSION I: FRAMING THE NEED FOR CHANGE: ENVISIONING A CLINICAL TRIALS ENTERPRISE IN THE HEALTH CARE SYSTEM OF 2020

Session Objectives:

- Set a framework within which to establish a vision for 2020 that addresses the reinvention of the clinical trials enterprise around partnership with the institutional health care delivery system of 2020.
- Given the anticipated changes in the structure of the U.S. health care system, consider how an efficient, effective clinical trials enterprise can be integrated into the health care delivery system of 2020.

ALASTAIR J.J. WOOD, Session Chair
Partner & Managing Director
Symphony Capital

9:10 a.m.  Presentation of Discussion Paper: The Clinical Trials Enterprise in the United States: A Call for Disruptive Innovation

ROBERT CALIFF
Director, Duke Translational Medicine Institute
Professor of Medicine
Vice Chancellor for Clinical and Translational Research
Duke University Medical Center

GARY FILERMAN
President
Atlas Health Foundation
Panel Discussion with Speakers: A Framework for the Clinical Trials Enterprise in the Health Care System of 2020

Objectives:

- Suggest and discuss the key components of a transformed clinical trials enterprise incorporated into the integrated health delivery system of 2020.
- Discuss the research outputs needed from the clinical trials enterprise to drive improved patient care and feed into a learning health system.
- Consider broad transformational changes including the changing shape/structure of the pharmaceutical industry and the increasing globalization in clinical trials and drug development.

Alastair J.J. Wood, Panel Moderator
Partner & Managing Director
Symphony Capital

Neil Weissman
President
MedStar Health Research Institute

Ihor Rak
Vice President, Clinical Neuroscience
AstraZeneca

10:45 a.m. BREAK

11:00 a.m. Keynote Address: The Learning Health System

Richard Platt
Professor, Harvard Medical School
Co-Chair, Clinical Effectiveness Research Innovation Collaboration
IOM Roundtable on Value and Science-Driven Health Care

11:30 a.m. Discussion with Keynote Speaker, Panelists, and Workshop Participants:

- How can the national clinical trials enterprise fit into the envisioned learning health system?
- What infrastructure is needed to embed randomized controlled trials in a learning health system?
- What are the key barriers impeding integration of clinical trials into a learning health system (e.g., financial, cultural, regulatory)?
Where are the opportunities to address these barriers and build the needed infrastructure? What stakeholders/organizations/sectors should take the lead and how can their efforts best be coordinated?

**Peter Yu, Moderator**
Oncologist, Palo Alto Medical Foundation
Chair, Electronic Health Record Working Group
American Society of Clinical Oncology

**Bryan Luce**
Senior Vice President, Science Policy
United BioSource Corporation

12:15 p.m. LUNCH

### SESSION II: DEVELOPING A ROBUST CLINICAL TRIALS WORKFORCE

**Session Objectives:**
- Define a clinical trials “workforce” and identify workforce needs that will be necessary to support clinical trials.
- Given the defined workforce, discuss the needs for career paths and career development opportunities.

**Sherine Gabriel, Session Chair**
William J. and Charles H. Mayo Professor of Medicine & Epidemiology
Mayo Medical School
Co-Principal Investigator and Director of Education, Mayo Clinical Center
for Translational Scientific Activities (CTSA)

1:15 p.m. Presentation of Discussion Paper: *Developing a Robust Clinical Trials Workforce*

**Robert Califf**
Director, Duke Translational Medicine Institute
Professor of Medicine
Vice Chancellor for Clinical and Translational Research
Duke University Medical Center

**Elaine Gallin**
Principal
QE Philanthropic Advisors

**Michael Lauer**
Director, Division of Cardiovascular Sciences
National Heart, Lung, and Blood Institute
National Institutes of Health
2:15 p.m.  **Panel Discussion with Speakers: Developing and Sustaining a Clinical Trials Workforce**

**Objectives:**
- List key skills, techniques, and areas of expertise needed by the workforce.
- What are the benchmarks and metrics of success for an effective clinical trials workforce?
- Propose and discuss the core competencies of a clinical trials workforce.

**Sherine Gabriel, Panel Moderator**
William J. and Charles H. Mayo Professor of Medicine & Epidemiology
Mayo Medical School
Co-Principal Investigator and Director of Education, Mayo Clinical Center for Translational Scientific Activities (CTSA)

**Briggs W. Morrison**
Senior Vice President, Worldwide Medical Excellence
Pfizer Inc.

**Rebecca Jackson**
Professor and Associate Dean for Clinical Research
Director and Principal Investigator, OSU Center for Clinical and Translational Science
The Ohio State University

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

3:15 p.m.  **Keynote Address: Sustaining Institutional Support and Patient Engagement in Clinical Trials: Models and Messages from the NIH Clinical Center**

**John Gallin**
Director
National Institutes of Health Clinical Center

3:45 p.m.  **Discussion with Keynote Speaker, Panelists, and Workshop Participants:**
- What are the needs for patient and broader public engagement in clinical trials?
- Discuss broad as well as disease-specific efforts to partner with patients in the development and conduct of clinical trials. How might these partnerships help create a national culture of clinical research in the United States?
- How can models and best practices from the NIH Clinical Center be adapted to the realities of other institutions and community systems to advance and promote the conduct of clinical trials in those systems?
JANET TOBIAS, Moderator
Co-founder and Partner, Ikana Health
Adjunct Assistant Professor, Mount Sinai School of Medicine

ANNETINE GELJINS
Professor of Health Policy
Co-Chair, Department of Health Evidence and Policy
Mount Sinai School of Medicine

HEATHER SNYDER
Senior Associate Director, Scientific Grants
Medical and Scientific Relations
Alzheimer’s Association

4:30 p.m. ADJOURN
Envisioning a Transformed Clinical Trials Enterprise in the United States: Establishing an Agenda for 2020

November 8, 2011

8:30 a.m. Breakfast available

9:00 a.m. Welcome and Introductions

JEFFREY DRAZEN, Workshop Chair
Editor-in-Chief
New England Journal of Medicine

SESSION III: ALIGNING CULTURAL AND FINANCIAL INCENTIVES

Session Objective:
- Consider institutional needs to support clinical trials including addressing cultural issues and aligning financial incentives to support and sustain an efficient clinical trials enterprise.

ARTHUR H. RUBENSTEIN, Session Chair
Professor, Department of Medicine, Division of Endocrinology
Raymond and Ruth Perelman School of Medicine
University of Pennsylvania

9:10 a.m. Presentation of Discussion Paper: Transforming the Economics of Clinical Trials

JUDITH KRAMER
Associate Professor of Medicine, Duke University Medical Center
Executive Director, Clinical Trials Transformation Initiative (CTTI)

KEVIN SCHULMAN
Professor of Medicine and Business Administration
Duke University School of Medicine and The Fuqua School of Business

9:50 a.m. Panel Discussion with Speakers: Aligning Cultural and Financial Incentives to Support Clinical Trials

Objectives:
- Discuss incentives, reward structures, and business models impacting the conduct of clinical trials, and the key needs and challenges for advancing and improving upon these models.
- Consider regulatory, financial, and cultural changes that are needed to improve the environment for clinical trials.
SESSION IV: BUILDING AN INFRASTRUCTURE TO SUPPORT A TRANSFORMED CLINICAL TRIALS ENTERPRISE

Session Objectives:
- Set out an organizational framework for an infrastructure to conduct clinical trials.
- Discuss:
  - organizational frameworks for the infrastructure (i.e., network models or other approaches);
  - informatics needs;
  - regulatory reform or harmonization both within the United States and internationally.

Clyde Yancy, Session Chair
Magerstadt Professor of Medicine
Chief, Division of Cardiology
Feinberg School of Medicine
Northwestern University

10:55 a.m. Presentation of Discussion Paper: Developing a Clinical Trials Infrastructure

Paul Eisenberg
Senior Vice President
Global Regulatory Affairs and Safety
Amgen Inc.
PETRA KAUFMANN  
Director, Office of Clinical Research  
National Institute of Neurological Disorders and Stroke  
National Institutes of Health

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

11:45 a.m.  **Panel Discussion with Speakers: Establishing an Infrastructure to Support a Transformed Clinical Trials Enterprise**

*Objectives:*

- Consider the types of trials that should be done in the United States and what the site/locus for the conduct of these trials should be within the infrastructure model proposed.
- What are the roles and responsibilities of the various participants and stakeholders in the proposed infrastructure of the clinical trials enterprise?

**CLYDE YANCY, Panel Moderator**  
Magerstadt Professor of Medicine  
Chief, Division of Cardiology  
Feinberg School of Medicine  
Northwestern University

**LOUIS FIORE**  
Principal Investigator, VA Cooperative Studies Program  
Director, VA Cooperative Studies Program Coordinating Center, Boston  
Department of Veterans Affairs

**MICHAEL KING JOLLY**  
Senior Vice President  
Quintiles Innovation

**KAREN MARGOLIS**  
Professor of Medicine, University of Minnesota Medical School  
Director of Clinical Research  
HealthPartners Research Foundation

12:30 p.m.  **LUNCH**

**SESSION V: DEVELOPING AN AGENDA FOR THE CREATION OF A TRANSFORMED CLINICAL TRIALS ENTERPRISE**

*Session Objectives:*

- Define the key steps and responsible parties needed to achieve the elements of a transformed clinical trials enterprise discussed during the workshop.
• Consider the prioritization of transforming different elements of the clinical trials enterprise—what is feasible and most desirable?
• Consider any unintended consequences of transforming the clinical trials enterprise.
• Suggest a transformative path forward for decision makers and those implementing the work of clinical trials across the enterprise.

1:30 p.m.  
JEFFREY DRAZEN, Session Chair  
Editor-in-Chief  
New England Journal of Medicine

Presentation of Key Themes/Suggested Paths from Session Chairs

ALASTAIR J.J. WOOD, Session I Chair  
Partner & Managing Director  
Symphony Capital

SHERINE GABRIEL, Session II Chair  
William J. and Charles H. Mayo Professor of Medicine & Epidemiology  
Mayo Medical School  
Co-Principal Investigator and Director of Education, Mayo Clinical Center for Translational Scientific Activities (CTSA)

ARTHUR H. RUBENSTEIN, Session III Chair  
Professor, Department of Medicine, Division of Endocrinology  
Raymond and Ruth Perelman School of Medicine  
University of Pennsylvania

CLYDE YANCY, Session IV Chair  
Magerstadt Professor of Medicine  
Chief, Division of Cardiology  
Feinberg School of Medicine  
Northwestern University

2:30 p.m.  
Reflecting on Potential Paths Forward: Setting an Agenda for a Transformed Clinical Trials Enterprise

JEFFREY DRAZEN, Panel Moderator  
Editor-in-Chief  
New England Journal of Medicine

DOUGLAS CROPPER  
President and Chief Executive Officer  
Genesis Health System

LYNN ETHEREDGE
Co-Founder
Health Insurance Reform Project and Rapid Learning Project
George Washington University

RONALD KRALL
Associate Fellow
University of Pennsylvania, Center for Bioethics

JAMES DOROSHOW
Director, Division of Cancer Treatment and Diagnosis
National Cancer Institute, National Institutes of Health

JEAN ROULEAU
Scientific Director
Institute for Circulatory and Respiratory Health
Canadian Institutes of Health Research (CIHR)

4:30 p.m. ADJOURN