INSTITUTE OF MEDICINE
OF THE NATIONAL ACADEMIES

Forum on Drug Discovery, Development, and Translation
Forum on Neuroscience and Nervous System Disorders
National Cancer Policy Forum
Roundtable on Translating Genomic-Based Research for Health

Sharing Clinical Research Data: An Institute of Medicine Workshop

AGENDA

October 4 - 5, 2012
National Academy of Sciences Building, Room 125
2101 Constitution Avenue, N.W.
Washington, DC

Background:

Pharmaceutical companies, academic institutions, advocacy organizations, and government agencies such as FDA and NIH have large quantities of clinical research data. Increased data sharing could facilitate scientific and public health advances, among other potential benefits to patients and society. Much of this information, however, is not transparent or shared beyond the data owner. More specifically, study results are not always published and where results are published, they typically only include summary-level data; participant-level data is privately held and rarely shared or revealed publicly.

This workshop will explore the benefits of and barriers to the sharing of clinical research data and will help identify strategies for enhancing sharing both within and across sectors. To facilitate identification of key issues and potential solutions, the workshop will focus on data resulting from preplanned interventional studies of human subjects. While recognizing the importance of other data sources such as observational studies and electronic health records, this focus was selected to encourage concrete problem-solving discussions over the course of a day-and-a-half meeting. Models and projects that involve sharing other types of data will be considered during the workshop to the extent that these models provide lessons and best practices applicable to sharing preplanned interventional clinical research data.

The workshop is being jointly organized by the Institute of Medicine's Forum on Drug Discovery, Development, and Translation; Forum on Neuroscience and Nervous System Disorders; National Cancer Policy Forum; and Roundtable on Translating Genomic-Based Research for Health.

Meeting Objectives:

- Examine the benefits of sharing of clinical research data, and specifically clinical trial data, from all sectors and among these sectors, including, for example:
  - Benefits to the research and development enterprise
  - Benefits to the analysis of safety and efficacy
- Identify barriers and challenges to sharing clinical research data
- Explore strategies to address these barriers and challenges, including identifying priority actions and "low-hanging fruit" opportunities
- Discuss strategies for using these potentially large data sets to facilitate scientific and public health advances.

Day One

8:30 a.m.   Opening Remarks

SHARON TERRY, *Workshop Chair*
President and Chief Executive Officer
Genetic Alliance

SESSION I: BENEFITS OF SHARING CLINICAL RESEARCH DATA

Session Objectives:
- Provide an overview of the benefits of sharing clinical research data, specifically clinical trial data, and discuss advantages and disadvantages of sharing participant vs. summary level data from individual trials as well as pooling data across multiple studies.
- Consider examples of scientific success stories that illustrate what can be accomplished when clinical trial data is shared.

8:40 a.m.   Background and Session Objectives

WILLIAM POTTER, *Session Co-Chair*
Co-Chair Emeritus
Neuroscience Steering Committee
FNIH Biomarkers Consortium

DEBORAH ZARIN, *Session Co-Chair*
Director, ClinicalTrials.gov
National Library of Medicine
National Institutes of Health

8:50 a.m.   **Fundamentals and Benefits of Sharing Participant-Level Clinical Trial Data**

ELIZABETH LODER
Clinical Epidemiology Editor
British Medical Journal

9:10 a.m.   **Pooling Data from Multiple Clinical Trials to Answer Big Questions**

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

9:30 a.m.   **Panel Discussion: Perspectives on the Benefits of Sharing**
                 Clinical Trial Data
- Data sharing – what does it mean from your perspective?
- Considering the benefits and risks of sharing clinical research data, how extensively should it be shared to maximize new knowledge and ultimately patient benefit?

Panelists

HARLAN KRUMHOLZ
Harold H. Hines, Jr. Professor of Medicine and Epidemiology and Public Health
Yale University School of Medicine

MYLES AXTON
Editor
Nature Genetics

JESSE BERLIN
Vice President of Epidemiology
Janssen Research and Development, LLC.,
a Johnson and Johnson pharmaceutical company

Panel Moderators

WILLIAM POTTER, *Session Co-Chair*
Co-Chair Emeritus
Neuroscience Steering Committee
FNIH Biomarkers Consortium

DEBORAH ZARIN, *Session Co-Chair*
Director, ClinicalTrials.gov
National Library of Medicine
National Institutes of Health

10:30 a.m.   BREAK

SESSION II: DATA SHARING MODELS: DESIGN, BEST PRACTICES, AND LESSONS LEARNED

Session Objectives:
- Present examples, best practices, and lessons learned from projects across the continuum of data sharing opportunities (e.g., rapid publication of participant-level data, increased access to participant-level data for qualified researchers, or maximizing the use of clinical research data that is currently held in centralized locations by requiring sharing or access to subsets of data).

- Distill best practices and lessons learned that can be applied broadly to new projects to maximize the use of data from individual trials and/or data pooling initiatives.

10:45 a.m.   Background and Session Objectives

JEFFREY NYE, *Session Chair*
Vice President and Head
Neuroscience External Innovation
Janssen Research and Development, LLC.,
a Johnson and Johnson pharmaceutical company

10:55 a.m.   ***The Limits of Summary Data Reporting: Lessons from ClinicalTrials.gov***

DEBORAH ZARIN
Director, ClinicalTrials.gov
National Institutes of Health

11:10 a.m.   ***Models that Increase Access and Use of Data from Individual Clinical Trials***

The DataSphere Project

CHARLES HUGH-JONES
Vice President, Medical Affairs North America
Sanofi Oncology, on behalf of the Life Sciences Consortium
CEO Roundtable on Cancer

Yale/Medtronic Experience

RICHARD KUNTZ
Senior Vice President
Chief Scientific, Clinical and Regulatory Officer
Medtronic, Inc.

11:40 a.m.   ***Models that Foster Pooling and Analysis of Data***

FNIH Biomarkers Consortium Adiponectin Project

JOHN WAGNER
Vice President, Clinical Pharmacology
Merck & Co., Inc.

Novel Methods Leading to New Medications in Depression and Schizophrenia (NEWMEDS) Consortium

JONATHAN RABINOWITZ
Academic Lead, NEWMEDS Work Package on Advanced Data Analysis Techniques
Bar Ilan University

12:10 p.m.   *Series of Brief Presentations on Overcoming Challenges Facing Clinical Trial Data Sharing*

Challenge #1: Permissions

JENNIFER GEETTER
Partner
McDermott Will & Emery

Challenge #2: Techniques and Methodologies

JOHN IOANNIDIS *[via video conference]*
C.F. Rehnborg Chair in Disease Prevention
Stanford University

Challenge #3: Culture

KELLY EDWARDS
Acting Associate Dean, The Graduate School
Associate Professor, Bioethics and Humanities
University of Washington

12:40 p.m.   Discussion among speakers, panelists, and audience
Discussant:
- Sally Okun, Health Data Integrity & Patient Safety, PatientsLikeMe

Discussion Moderator

JEFFREY NYE, *Session Chair*
Vice President and Head
Neuroscience External Innovation
Janssen Research and Development, LLC.,
a Johnson and Johnson pharmaceutical company

1:00 p.m.   LUNCH

KEYNOTE CASE STUDY: DISTRIBUTED SYSTEMS FOR CLINICAL RESEARCH INFORMATION SHARING

1:30 p.m.   RICHARD PLATT
            Professor and Chair
            Department of Population Medicine
            Harvard Pilgrim Health Care Institute and Harvard Medical School

1:50 p.m.   Discussion with speaker and audience

            Discussion Moderator

            SHARON TERRY, *Workshop Chair*
            President and Chief Executive Officer
            Genetic Alliance

SESSION III: STANDARDIZATION AND GOVERNANCE

Session Objectives:
- Receive an update on recent legislative and regulatory language regarding standardization of clinical research data and discuss how stakeholders are designing and implementing data standardization plans in response.
- Discuss the relative cost-benefit of data conversion of existing trial data versus building an infrastructure to improve data collection and sharing moving forward.
- Present case studies from data sharing projects using different data standardization and governance models and consider lessons learned or best practices for the future.

2:00 p.m.   Background and Session Objectives

            FRANK ROCKHOLD, *Session Co-Chair*
            Senior Vice President, Global Clinical Safety and Pharmacovigilance
            GlaxoSmithKline Pharmaceuticals Research and Development

            LYNN HUDSON, *Session Co-Chair*
            Chief Science Officer and Executive Director
            Coalition Against Major Diseases
            Critical Path Institute

2:10 p.m.   **PDUFA Update on Data Standards**

            RON FITZMARTIN
            Senior Advisor, Office of Planning and Analysis
            Center for Drug Evaluation and Research
            U.S. Food and Drug Administration

2:25 p.m.   *Standardization to Facilitate Data Sharing: Opportunities and Limitations*

                   CDISC Efforts to Support Clinical Research Data

                   REBECCA KUSH
                   President and Chief Executive Officer
                   Clinical Data Interchange Standards Consortium

                   HL7 Efforts to Support Clinical Care Data

                   CHARLES JAFFE
                   Chief Executive Officer
                   Health Level 7 International

                   Health Information Technology Perspective on Clinical Research Data Standards

                   SACHIN JAIN
                   Chief Medical Information and Innovation Officer
                   Merck & Co., Inc.

3:10 p.m.   Discussion with speakers and audience

3:30 p.m.   BREAK

3:45 p.m.   **Cost-Benefit Analysis of Retrospective vs. Prospective Data Standardization**

                   VICKI SEYFERT-MARGOLIS
                   Senior Advisor, Science Innovation and Policy
                   Office of the Chief Scientist
                   U.S. Food and Drug Administration

4:00 p.m.   *Case Studies: Standardization and Governance Models in Data Sharing*

                   Critical Path Institute and Coalition Against Major Diseases (CAMD) Alzheimer's Clinical Trial Database

                   CAROLYN COMPTON
                   President and CEO
                   Critical Path Institute

                   Translational Medicine Mart (tranSMART)

                   ERIC PERAKSLIS
                   Chief Information Officer and Chief Scientist, Informatics
                   U.S. Food and Drug Administration

4:30 p.m.   *Panel Discussion*
- ▪ Catalog new data sharing challenges not yet discussed and provide suggestions for overcoming these challenges.
- ▪ Given the data standardization and governance models discussed, suggest a framework to guide the development of new data sharing projects based on their purpose (e.g., regulatory approval with FDA, detecting safety signals, testing secondary hypotheses, etc.)

Panelists

LAURA LYMAN RODRIGUEZ
Director
Office of Policy, Communications and Education
National Human Genome Research Institute

MEREDITH NAHM
Associate Director for Clinical Research Informatics
Duke Translational Medicine Institute

NEIL DE CRESCENZO
Senior Vice President and General Manager
Oracle Health Sciences

MICHAEL CANTOR
Senior Director
Clinical Informatics and Innovation
Pfizer Inc.

Panel Moderators

FRANK ROCKHOLD, *Session Co-Chair*
Senior Vice President, Global Clinical Safety and Pharmacovigilance
GlaxoSmithKline Pharmaceuticals Research and Development

LYNN HUDSON, *Session Co-Chair*
Chief Science Officer and Executive Director
Coalition Against Major Diseases
Critical Path Institute

5:30 p.m.   Adjourn day 1

Day Two

8:00 a.m.   Opening Remarks

SHARON TERRY, *Workshop Chair*
President and Chief Executive Officer
Genetic Alliance

SESSION IV: INCENTIVIZING POLICY AND CULTURAL SHIFTS TO ENHANCE DATA SHARING

Session Objectives:
- Receive an update on clinical trial data transparency decisions in Europe.
- Explore current incentives for and against (i.e., benefits and risks of) data sharing within and across sectors and suggest mechanisms to encourage stakeholders to engage in a culture of data sharing.
- Identify existing and potential strategies, including technology-based approaches, for protecting patient privacy and confidentiality while facilitating data sharing.

8:10 a.m.   Background and Session Objectives

ROBERT HARRINGTON, *Session Chair*
Arthur L. Bloomfield Professor of Medicine
Chair, Department of Medicine
Stanford University

8:20 a.m.   *Clinical Trial Data Transparency: European Medicines Agency Perspective*

HANS-GEORG EICHLER
Senior Medical Officer
European Medicines Agency

8:40 a.m.   *Clinical Research Data Sharing Practices and Attitudes*

ANDREW VICKERS
Attending Research Methodologist
Department of Epidemiology and Biostatistics
Memorial Sloan-Kettering Cancer Center

8:55 a.m.   ***Overview of Data Sharing Policies: Research Funders and Publishers***

STEVEN GOODMAN
Associate Dean for Clinical and Translational Research
Professor of Medicine & Health Research and Policy
Stanford University School of Medicine

9:10 a.m.   ***Series of Presentations: Incentives for Data Sharing Within and Across Sectors***

Academic Perspectives

PETER DOSHI
Post-Doctoral Fellow
Johns Hopkins University School of Medicine

BETH KOZEL
Instructor of Pediatrics
Division of Genetics and Genomic Medicine
St. Louis Children's Hospital and Washington University School of Medicine

Federal Research Funder Perspective

JOSEPHINE BRIGGS
Director, National Center for Complementary and Alternative Medicine
Director, National Center for Advancing Translation Sciences, Division of Clinical Innovation
National Institutes of Health

9:55 a.m.   Discussion with speakers and audience

10:30 a.m.   BREAK

10:45 a.m.   ***Facilitating Patient Ownership of Clinical Trial Data: Technical Challenges and Opportunities***

JOHN WILBANKS
Director
Sage Bionetworks

DEVEN MCGRAW
Director, Health Privacy Project
Center for Democracy and Technology

11:15 a.m.   Discussion with speakers and audience

SESSION V: NEXT STEPS AND FUTURE DIRECTIONS

Session Objectives:
- Discuss key themes from the workshop.
- Based on workshop presentations and discussions, identify potential next steps and priority actions for data sharing stakeholders to take action.
- Highlight potential opportunities and challenges that are currently on the horizon but may become more salient as technology evolves and/or data sharing becomes more pervasive.

11:30 a.m.   Background and Session Objectives

SHARON TERRY, *Workshop Chair*
President and Chief Executive Officer
Genetic Alliance

11:40 a.m.   ***Session Chair Reports*** *[5 minutes per Session]*

WILLIAM POTTER, *Session I Co-Chair*
Co-Chair Emeritus
Neuroscience Steering Committee
FNIH Biomarkers Consortium

DEBORAH ZARIN, *Session I Co-Chair*
Director, ClinicalTrials.gov
National Library of Medicine
National Institutes of Health

JEFFREY NYE, *Session II Chair*
Vice President and Head
Neuroscience External Innovation
Janssen Research and Development, LLC.,
a Johnson and Johnson pharmaceutical company

FRANK ROCKHOLD, *Session III Co-Chair*
Senior Vice President
Global Clinical Safety and Pharmacovigilance
GlaxoSmithKline Pharmaceuticals Research and Development

LYNN HUDSON, *Session III Co-Chair*
Chief Science Officer and Executive Director
Coalition Against Major Diseases, Critical Path Institute

ROBERT HARRINGTON, *Session IV Chair*
Arthur L. Bloomfield Professor of Medicine
Chair, Department of Medicine
Stanford University

12:00 p.m.   ***Closing Discussion with Session Chairs, Panelists, and Audience Led by Workshop Chair***

JOSEPHINE BRIGGS
Director, National Center for Complementary and Alternative Medicine
Director, National Center for Advancing Translation Sciences, Division of Clinical Innovation
National Institutes of Health

MICHAEL ROSENBLATT
Executive Vice President and Chief Medical Officer
Merck & Co., Inc.

JAY "MARTY" TENENBAUM
Founder and Chairman
Cancer Commons

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

12:45 p.m.   Adjourn