

***New Paradigms in Drug Discovery: How Genomic Data are
Being Used to Revolutionize the Drug Discovery and
Development Process – A Workshop***

March 21, 2012

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

Workshop Objective:

- To examine the impact of and investment in the use of genetic and genomic data in drug development.
 - To discuss how genomic and genetic data has been and will be used in the drug development process to improve aspects such as target identification, clinical trial design, pharmacogenomics approaches, biomarker development and understanding of disease biology.
 - To investigate the economic drivers, incentives, and models that use genomics in drug development.
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7:45 A.M. Working Breakfast

8:30 A.M. Welcoming remarks

Wylie Burke, *Roundtable Chair*
Professor and Chair
Department of Bioethics and Humanities
University of Washington

8:35 A.M. Charge to workshop speakers and participants

Geoffrey Ginsburg
Director, Center for Genomic Medicine
Duke University

SESSION I: CURRENT LANDSCAPE

Session Focus: Current use and utility of genomics-guided drug development strategies

Session Questions: What has been the impact and what is expected to be the potential impact of genomics on drug development?
What is the current investment?
What are the challenges in using genomics information to further the understanding of the underlying basis of disease and identification of drug targets?
What are the economics and incentives/disincentives for utilizing genomics in drug development?
Can we see examples where genomics drives positive economic benefit and where there's a negative impact? What is different between these situations?
What does the overall landscape of drug development and its challenges look like over the next 5 years? How will genomics play into that landscape? Where are the real opportunities for positive impact? What structural and economic forces are likely to be at play and how might they impact upon productivity in the industry?
What challenges are there in developing biomarkers for translational purposes in drug development? Where will we see them be a success?

Moderator: Aidan Power, Pfizer Inc.

8:45 A.M. **Current use of genetic and genomic strategies in drug development**

Nicholas Davies
Partner, Pharmaceutical and Life Sciences Practice
PwC

9:00 A.M. **Economic incentives for genetic and genomic strategies**

Mark Trusheim
Visiting Scientist and Executive-in-Residence, MIT Sloan School of Management; President, Co-Bio Consulting

9:15 A.M. **Perceived challenges in genomic-based drug development**

Garret A. FitzGerald
Professor of Medicine & Pharmacology and McNeil Professor in
Translational Medicine & Therapeutics; Associate Dean for
Translational Research; Chair, Department of Pharmacology;
Director Institute for Translational Med. & Therapeutics
University of Pennsylvania School of Medicine

9:30 A.M. **Discussion with speakers and attendees**

10:15 A.M. BREAK (15 minutes)

SESSION II: DRUG DISCOVERY AND DEVELOPMENT

Session Focus: Genomics-enabled target identification and drug development

Session Questions: What was the rationale/driver behind including genomics in the drug program?
How and when did the genomic hypothesis evolve?
At which point from concept to registration did genomics become completely embedded into the drug development program and how did it influence the process?
What were the biggest challenges in having a genomics based drug development program as compared to the standard paradigm?
How did the research and development and commercial teams align?
What are the challenges and potential solutions for aligning validation approaches to meet various global regulatory agency requirements?

Moderator: Michelle Penny, Eli Lilly and Company

10:30 A.M. **Case studies of genomics-based drug development**

Development of Crizotinib for treatment of non-small cell lung cancer

Steffan N. Ho
Director, Translational Oncology
Pfizer Inc.

Use of Genetics to Inform Drug Development of a Novel Treatment for Schizophrenia

Laura Nisenbaum
Senior Research Advisor, Pharmacogenomics
Translational Medicine and Tailored Therapeutics
Eli Lilly and Company

A Genetic Approach to the Treatment of Cystic Fibrosis

Peter Mueller
Executive Vice President, Global Research and Development
Chief Scientific Officer
Vertex Pharmaceuticals Incorporated

11:30 A.M. **Discussion with speakers and attendees**

12:15 P.M. **WORKING LUNCH**

SESSION III: EMERGING TECHNOLOGIES

Session Focus: Current application of emerging technologies, such as next-generation sequencing, to drug discovery and development

Session Questions: Where are genomics technologies now standard in the drug development process? And where can new technologies add value to the current drug development paradigm?

What is the process for evaluating the benefit and impact of novel or emerging technologies in drug discovery and development?

What are the challenges to introducing and using novel genomic technologies (such as NGS) in the context of drug discovery? And in particular in clinical development and clinical trials?

How should we address the challenges and limitations of applying genomic technologies in drug development?

How do you see the development of a genomic-based diagnostic being coordinated with a novel therapeutic for approval? Would there be any modifications to the current process of clinical development that would need to be undertaken for this to happen?

What are some of the early examples of successful implementation of genomic technologies in facilitating approval of a drug or getting a drug to market? What are the lessons learned from these examples?

Moderator: Geoffrey Ginsburg, Duke University

1:00 P.M. **Utility of emerging genomics technology in drug development**

Complete genomics

Radoje Drmanac
Cofounder and Chief Scientific Officer

Foundation Medicine

Gary Palmer
Senior Vice President, Medical Affairs and Commercial
Development

Genentech

Jane Fridlyand
Senior Statistical Scientist

1:45 P.M. **Discussion with speakers and attendees**

SESSION IV: EVOLVING PARADIGMS

Session Focus: Partnerships in genomics- and biomarker-enabled drug development, approval and prescribing

Session Questions: Using your experience and company/foundation/agency as an example, and as a jumping off point for the more general issues relative to paradigms:

What are the limitations you have experienced in moving foundation-driven research into pharmaceutical company development?

What would accelerate drug development from a foundation's point of view?

What do foundations need to accelerate drug development?

What are the incentives for entering into partnerships with academia – beyond the obvious, how would you characterize strengths and weaknesses and challenges?

How does genomic information give strength and leverage to company-academic partnerships – what is unique about genomics than can accelerate a paradigm change?

How does regulatory policy influence the use and utility of genomics-based drug development?

What have been the effects of the Voluntary Exploratory Data Submissions (VXDS) Program? What would improve that program?

How can genomic information be leveraged to identify new indications for existing pharmaceuticals?

What have you tried in the repurposing arena? What has worked? What has failed?

What story does Medco data tell about the use and benefit of pharmacogenomics?

How can pharmacy benefit management accelerate drug development – give examples of successes and failures.

What do you wish you could try – and why can't you?

Moderator: Sharon Terry, Genetic Alliance

2:15 P.M. Foundations and drug development

Walter Capone
Chief Operating Officer
Multiple Myeloma Research Foundation

Genomics and regulatory science

Michael Pacanowski
Team Leader
Office of Clinical Pharmacology
Office of Translational Sciences
Center for Drug Evaluation and Research
U.S. Food and Drug Administration

2:45 P.M. BREAK

3:00 P.M. Repurposing of drugs

Christopher Austin
Director of the Division of Pre-Clinical Innovation
Scientific Director, NIH Center for Translational Therapeutics
National Center for Advancing Translational Sciences
National Institutes of Health

Pharmacy benefit management and pharmacogenomics

Felix W. Frueh
President
Medco Research Institute

3:30 P.M. **Discussion with speakers and attendees**

**SESSION V: LEADING THE STRATEGY FOR PERSONALIZED MEDICINE: PHARMA,
GOVERNMENT, ACADEMIA – HOW DO WE ALL WORK TOGETHER**

Session Focus: A discussion of benefits and limitations of adopting a genomics-based approach to drug development

Session Questions: Has genomic information fundamentally changed drug discovery and development?
Where have expectations for genomics fallen short?
What is the potential for genomic information to alter the current paradigm?
What will the future of drug development look like and what is necessary to achieve this goal?
With increasing calls for pre-competitive collaborations, in what ways might NCATS, FDA and the new Regulatory Science Initiative work together to stimulate novel drug discovery and development research?
Can you foresee the need for an American public-private partnership organization similar in scope and mission to the European Innovative Medicines Initiative?
What challenges might NCATS, FDA and the Regulatory Science Initiative face in catalyzing more rapid genomic-based drug discovery and development within the current legal regulatory environment?
How can the significant investments that have been made in academic research be leveraged in an environment of limited resources? In a global environment?

Moderator: Thomas Lehner, National Institute of Mental Health

4:15 P.M. **Discussants**

Deborah Dunsire
President and CEO
Millennium: The Takeda Oncology Company

Victor Dzau
Chancellor for Health Affairs
Duke University
President and CEO
Duke University Health System

Margaret Hamburg
Commissioner
U.S. Food and Drug Administration

Kathy Hudson
Deputy Director for Science, Outreach, and Policy
Acting Deputy Director, National Center for Advancing
Translational Research
National Institutes of Health

5:30 P.M. **Concluding Remarks**

5:45 P.M. **Adjourn**