



NIH's Francis Collins with HHS Secretary Kathleen Sibelius



President Barack Obama at the National Academies

ABOUT THE FORUM

The Forum on Drug Discovery, Development, and Translation was created in 2005 by the Institute of Medicine's Board on Health Sciences Policy to provide an opportunity for stakeholders to meet and discuss issues of mutual interest in a neutral setting. It includes leaders from the pharmaceutical and biotech industries, academia, federal agencies, foundations, and patient groups. The Forum convenes several times each year to identify and discuss key problems and strategies in the discovery, development, and translation of drugs. It commissions papers, convenes workgroups, and fosters collaborations among its members and constituencies. In order to engage a wide range of experts, members of the public, and the policy community in discussing specific issues of concern in the science and policy of drug development, the Forum holds regular public workshops and symposia. Proceedings and speaker presentations are disseminated to the public through published summaries and the Forum website www.iom.edu/drug.



FDA Commissioner swearing-in ceremony for Margaret Hamburg



FDA Community Update Meeting at the NAS Keck Building

MESSAGE FROM THE CO-CHAIRS

The year 2009 saw continuing upheaval in the scientific and business landscape of drug development. The process of discovering and developing new drugs is more dynamic, more global, and more risky than ever. But these developments were overshadowed by political events, specifically the change in administrations in Washington, D.C., which has brought significant change to the scientific landscape and priorities of the nation—from stem cell research to comparative effectiveness research to health care reform.



leff Drazen

With the new administration came a renewed emphasis on science, as expressed by President Obama in a speech he delivered at the National Academy of Sciences, "Science is more essential for our prosperity, our security, our health, our environment, and our quality of life than it has ever been before."

For drug development, the appointment of new leadership at the NIH and the FDA is especially exciting and holds the promise of innovative and collaborative approaches to drug development and regulation. The new NIH director has made drug development for orphan diseases as one of his top priorities. This focus will undoubtedly lead to heightened interest in all phases of drug discovery and development in academia. The activities of the Forum in 2009 reflected the excitement and dynamism of this new environment, focusing on four critical areas:

- 1. PROMOTING THE SCIENTIFIC BASIS FOR THE REGULATION OF DRUGS
- 2. TRANSFORMING CLINICAL RESEARCH

al I. Casell

- 3. FOSTERING A ROBUST RESEARCH ENVIRONMENT THROUGH COLLABORATION
- 4. PROMOTING GLOBAL LEADERSHIP IN DRUG DEVELOPMENT AND TRANSLATION

This was a year to remember for new ground covered. The Forum provided an important lens for its diverse members and the public to collectively reflect, understand, and act on this changing environment. We look forward and hope for an equally exciting and promising 2010.

Gail Cassell

Jeff Drazer Co-chair

PROMOTING THE SCIENTIFIC BASIS FOR THE REGULATION OF DRUGS

The Forum held a series of public meetings on drug safety throughout 2009. The first addressed personalized medicine and the genetic basis of adverse events. The second featured the new FDA commissioner, Peggy Hamburg, and her vision for the FDA. The third



IOM President Harvey Fineberg and FDA Commissioner Peggy Hamburg

focused on advances in post-market drug safety since the passage of FDAAA. A fourth meeting, to be held in 2010, will focus on the science infrastructure at FDA and the establishment of centers of excellence to advance regulatory science. A subsequent meeting will address the assessment of therapeutic benefits and risks in decision-making at FDA.

HISTORY OF THE DRUG FORUM

Since 2007, the Forum has hosted public meetings designed to engage congressional staff and the broader policy community on current topics in drug development. These sessions are designed to provide concise, balanced, and insightful overviews of current issues—including both science and policy implications—in a neutral, advocacy-free setting. Proceedings are not published, but presentations are available on the Forum website.

FDA Community Update: Personalized Medicine and the Genetic Basis of Adverse Events

Following the publication of the IOM report on the FDA, *The Future of Drug Safety: Promoting and Protecting the Health of the Public*, and the passage of the FDA Amendments Act of 2007, the Forum initiated the FDA Community Update discussion series to keep the Washington policy community apprised of the agency's progress on implementing its safety initiatives. In March 2009, the Forum invited speakers from the FDA, academia, industry, and major policy think tanks to update the public on the FDA's work on personalized medicine, and to consider the potential policy challenges for the future in pharmacogenomics.

FDA Community Update: Postmarket Drug Safety



Joanne Meyer, Novartis

In September 2009, the Forum gathered experts from various sectors to address the agency's progress in postmarket safety science. Speakers provided updates on the Sentinel Initiative, Risk Evaluation and Mitigation Strategies, Observational Medical Outcomes Project, and other safety initiatives mandated by FDAAA legislation two years ago. In an interactive discussion with the audience, the speakers emphasized the need for correct and urgent implementation of postmarket initiatives, and outlined possible policy issues that may rise in the FDA's safety-oriented environment.

March – Co-Chair Gail Cassell and Ed Holmes convene the first meeting of the Drug Forum

TRANSFORMING CLINICAL RESEARCH

The Forum recognizes that the critical link between scientific discovery and medical utility is clinical trials—testing whether or not a new therapeutic product performs as expected and makes a difference in treating disease. To plan and execute a clinical trial today can take years and cost hundreds of millions of dollars. To engage stakeholders in an honest discussion of the state of clinical trials in the United States today and gain an understanding of the challenges to conducting clinical trials, the Forum held the first in a series of workshops in October 2009. Workshop participants identified areas of strength and weakness in our current clinical trials enterprise, and considered transformative strategies for enhancing the ways in which clinical research is organized and conducted. The next workshop, planned for 2010, will address proposals for a national clinical research infrastructure in the United States to effectively bridge the divide between clinical research and clinical practice. The Forum will also bring this important discussion to a broader audience of policymakers, practicing clinicians, academic researchers, and patients across the United States through a sub-series of regional meetings on clinical research.

May-Workshop on Understanding the Benefits and Risks of Pharmaceuticals



Forum member Robert Califf

Transforming Clinical Research in the United States

The Forum launched a series of workshops to examine the state of clinical trials in the United States. The workshop series seeks to identify strengths and weaknesses in the existing clinical trials enterprise, and consider transformative strategies for enhancing the ways in which clinical research is organized and conducted. The first workshop, held in October 2009, examined four disease areas—cardiovascular disease, depression, cancer, and diabetes—to explore how clinical research approaches vary by disease, and to identify lessons

from these examples that can be applied to clinical research in other disease areas. Presentations are available on the Forum website, and a workshop summary will be released in 2010.



Depression Panel at the Clinical Trials Workshop: William Potter, Merck; Madhukar Trivedi, Southwestern Medical Center; lim McNulty, Depression and Bipolar Disorder Alliance; Amir Kalali, Quintiles

March – Symposium on the IOM Drug Safety Report and Challenges for the FDA

2007

September–Biomedical Science and Policy Discussion Series, "Patient-Driven Drug Discovery and Development"

FOSTERING A ROBUST RESEARCH ENVIRONMENT THROUGH COLLABORATION

In April, the Forum held a public meeting to explore ways to streamline the process by which universities, industry, and government negotiate agreements for research collaboration. The Forum commissioned a paper, which resulted in the development of material transfer agreement (MTA) and clinical trial agreement (CTA) templates that could be used to facilitate such negotiations. The NIH National Center for Research Resources has disseminated these documents for use by collaborating institutions. In another initiative, the Forum established a precompetitive collaboration to better understand drug failures, explore standards for data collection and analysis, and to ultimately foster improved decision-making with respect to research and investments in drug development. Member companies have begun to share and analyze information on late-stage attrition. Lastly, following the IOM's report on conflict of interest, the Forum began plans for a workshop in 2010 that will explore approaches to implementing the conflict of interest guidelines in a way that promotes an open and fair exchange among academic and commercial partners to advance human health.

February – Discussion Series Meeting on Comparative Effectiveness

June-Workshop on Breakthrough
Business Models for Drug Development

Material Transfer and Clinical Trial Agreements

In an effort to streamline the process of negotiations between universities, industry, and government, the Forum explored the use of material transfer agreement/clinical trial agreement (MTA/CTA) templates during a workshop in April 2009. A paper commissioned by the Forum resulted in a set of templates that have been widely-disseminated by NIH and others. They can be downloaded from the Forum website (www.iom.edu/CTA-MTA).



Forum Member Barbara Alving, NCRR



Susan Ehringhouse, AAMC, and Bernie Lo, UCSF

July – Jeff Drazen appointed co-chair

October–Workshop on Accelerating the Development of Biomarkers for Drug Safety

PROMOTING GLOBAL LEADERSHIP IN DRUG DEVELOPMENT AND TRANSLATION

The Forum's initiative on multidrug-resistant tuberculosis (MDR TB) includes a series of workshops quickly gaining international attention. The first workshops—held in the United States—presented new data on the magnitude of the MDR TB problem and the challenges to addressing the rapid spread of drug-resistant tuberculosis. These initial meetings led to the development of workshops to take place in the four highest burden countries—South Africa, Russia, China, and India. A workshop to be held in Moscow in May 2010 became the subject of negotiations for a Memorandum of Understanding between the Academies, NIH, and the Russian Academy of Sciences. The agreement was acknowledged by President Obama and Secretary Clinton during the President's visit to Moscow and led to collaboration between IOM and NIAID to develop coordinated MDR TB meetings in both Russia and South Africa in 2010.

February—Capitol Hill Briefing on Multidrug-Resistant Tuberculosis

April–Workshop on Streamlining Clinical Trial and Material Transfer Negotiations

The Global Threat of Multidrug-Resistant Tuberculosis (MDR TB)

Building on its November 2008 workshop on multidrug-resistant tuberculosis, *Addressing the Threat of Drug-Resistant Tuberculosis: A Realistic Assessment of the Challenges*, the Forum met on Capitol Hill in February 2009 to update members of Congress, their staffs, and policymakers on the global challenges of MDR TB. The meeting addressed growing problems in transmission and infection control, diagnosis, drug supply and quality, treatment, and the lack of new drug thera-



Paul Zintl, Partners-in-Health; Anthony Fauci, NIAID, NIH; Ken Castro, CDC

pies for fighting TB. Speakers addressed existing efforts identified leadership funding gaps, and discussed possible roles for the U.S. government. A published summary of the first workshop in November 2008, as well as presentations from both meetings, are now available on the Forum website. A series of follow-on workshops in the four most high-burden countries—South Africa, Russia, India, and China—are currently in development.



Michael Ugrumov, Russian Academy of Sciences, and Forum Co-chair, Gail Cassell



Paul Farmer, Partners in Health

September–FDA Community Update on Post-Market Drug Safety

INITIATIVES FOR 2010

A series of work group meetings will be held through 2010, in which information will be exchanged and analyzed, and strategies discussed.

Regulatory Science

The Forum will hold a public workshop in February 2010 to examine the state of the science of drug regulation and to consider approaches for enhancing the scientific basis of regulatory decision making. Individuals from the federal government, pharmaceutical industry, academia, industry, and patient groups will discuss the strengths and weaknesses of the current regulatory science framework, and consider a range of strategies for improving regulatory science, such as development of a scientific discipline of regulatory science, increased collaboration between regulatory and academic researchers, and enhancement of the regulatory science infrastructure.

Transforming Clinical Trials in the United States

In 2010, the Forum will continue its discussion on how clinical trials can be improved to enhance feedback to a learning health care system. The meeting will examine the need for a national infrastructure for clinical trials that will help to close the gap between clinical research and clinical practice. It will also consider alternative approaches and proposals for such an infrastructure, and consider strategies for improving the organization and design of clinical trials across different settings and clinical areas.

Conflict of Interest

Following the 2009 IOM report on conflict of interest, the Forum began the development of a public workshop to explore approaches to implementing the IOM's recommendations in ways that promote openness and collaboration among academia, industry and government. A key aspect of this workshop is a comparative analysis of approaches to managing conflicts in the United States and elsewhere, and a background paper is being commissioned in order to provide a baseline of information on international standards and best practices in managing conflict of interest. A workshop is planned for the Fall of 2010.

FDA Community Updates: Premarket Drug Safety and the Benefits and Risk of Pharmaceuticals

As a part of the Forum's broader Biomedical Science and Policy Discussion Series, the third FDA Community Update will examine progress made in the premarket drug safety system since the release of the IOM drug safety report and the passage of the FDA Amendments Act of 2007. The series will also consider the challenges and policy issues that remain. A fourth FDA Community Update will brief Washington policymakers on the agency's progress in communicating benefits and risks of pharmaceuticals to the medical community. The meeting will follow a 2006 Forum workshop on the same topic and the creation of new FDA advisory committees dedicated to risk communication and transparency issues.

The Growing Threat of Multidrug-Resistant Tuberculosis

In March and May of 2010, the Forum plans to hold MDR TB workshops in South Africa and Russia. The workshops will bring together local and global experts, as well government leaders, to open dialogue on overcoming the rising threat of MDR TB. Similar workshops in China and India are in development. The goal of these in-country meetings is to bring attention to the growing problem of MDR TB worldwide, and to consider alternative global strategies for transmission control, drug procurement, diagnosis, and treatment.

Drug Failures Analysis

The Forum established a precompetitive collaboration to better understand drug failures, explore the establishment of standards for data collection and analysis, and to ultimately foster better decision-making with respect to research and investments in drug development. While a number of IOM activities are exploring precompetitive collaboration from a conceptual and policy framework, this is an operational collaboration through which member companies have begun to share and analyze information in order to understand the root causes of late-stage attrition.

MEMBERS

(as of December 31, 2009)

Membership in the Forum includes a diverse range of stakeholders from multiple sectors, including government, the pharmaceutical and biotechnology industries, academic health centers, and patient groups.

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Editor-in-Chief, New England Journal of Medicine

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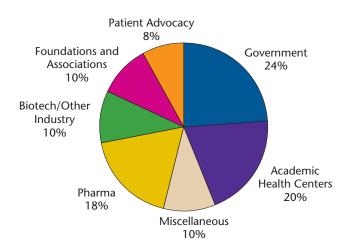
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ADDITIONAL INFORMATION

For more information about the Forum on Drug Discovery, Development, and Translation, please visit our web site at: www.iom.edu/drug.

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