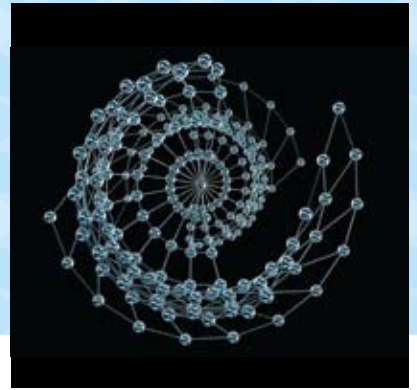


Forum on Drug Discovery, Development, and Translation

2010 Annual Report



INSTITUTE OF MEDICINE
OF THE NATIONAL ACADEMIES

Advising the nation • Improving health

Message from the Co-Chairs

Gail Cassell and Jeffrey Drazen

The Institute of Medicine's (IOM's) Forum on Drug Discovery, Development, and Translation was created in 2005 by the IOM's Board on Health Sciences Policy to foster dialogue among stakeholders and provide ongoing opportunities to discuss issues of mutual interest in a neutral setting. The Forum provides a venue for dialogue and collaboration among its membership, which includes leaders from the pharmaceutical and biotech industries, academia, federal agencies, foundations, and patient groups. The Forum brings ongoing attention and visibility to important issues in drug development; explores new approaches for resolving problem areas; helps define the scope of the field and thus sets the stage for future policy action; provides a catalyst for collaboration on topics where there is synergy among potential partners; and elevates the general understanding of drug discovery, development, and translation among the research, public policy, and broader communities. The Forum is self-governing, with Forum membership convening several times each year to identify and prioritize the topics they wish to address.

In 2010 the scientific and business landscape of drug development was fraught with continued uncertainty and risk. New paradigms for discovering and developing drugs are being sought to bridge the ever-widening gap between scientific discoveries and translation of those discoveries into life-changing medications. The landscape of the drug development enterprise is increasingly global, with an attending need to address cross-border issues in the regulatory, scientific, ethical, and economic arenas.

With such great challenges comes great opportunity and cause for optimism. The leadership at both the National Institutes of Health (NIH) and the Food and Drug Administration (FDA) have emphasized their support for and dedicated efforts to innovation in regulatory science and translational medicine. New collaborative approaches within the federal

agencies, academia, and industry are directing focused attention on the advancement of the drug development enterprise. Forum activities reflect this dynamic environment, focusing on five critical areas:

- Addressing the Approach to Drug Development: Problems and Opportunities
- Promoting and Enhancing the Scientific Basis for the Regulation of Drugs
- Transforming Research and Fostering Collaborative Research
- Developing Drugs for Rare and Neglected Diseases and Addressing Urgent Global Health Problems
- Promoting Public Understanding of Drug Development

We look forward to another groundbreaking and productive year for the Drug Forum in 2011.



A handwritten signature in black ink that reads "Gail H. Cassell".

Gail Cassell
Co-Chair



A handwritten signature in black ink that reads "Jeffrey M. Drazen MD".

Jeffrey Drazen
Co-Chair

Reflecting Back

Forum Activities in 2010

Forum Meetings

The Forum met three times in 2010. Discussions at these meetings focused on a diverse array of topics, including the needs for streamlining administrative inefficiencies and harmonizing regulatory responsibilities in the conduct of clinical trials and exploration of approaches to address societal and policy concerns about conflicts of interest in science and medicine. In addition, the Forum convened public workshops, described in detail below.

The Public Health Emergency Medical Countermeasures Enterprise—Workshop (February 2010)

During public health emergencies such as pandemic influenza outbreaks or terrorist attacks, effective vaccines, drugs, diagnostics, and other medical countermeasures are essential to protecting national security and the well-being of the public. The Public Health Emergency Medical Countermeasures Enterprise (PHEMCE)—a partnership among federal, state, and local governments; industry; and academia—is at the forefront of the effort to develop these countermeasures. At the request of the Secretary of the U.S. Department of Health and Human Services (HHS) and the Assistant Secretary for Preparedness and Response, the Drug Forum and the IOM’s Forum on Medical and Public Health Preparedness for Catastrophic Events collaborated to host a workshop, held February 22–24, 2010, to address challenges facing the PHEMCE. Workshop participants discussed federal policies and procedures affecting the research, development, and approval of medical countermeasures (MCMs) and explored opportunities to improve the process and protect Americans’ safety and health. The discussion at the workshop and workshop summary report helped inform the HHS Public Health Emergency Medical Countermeasures Enterprise Review, released in August 2010.

FORUM ACTIVITIES TIMELINE

2000–2004

2005

Clinical Research Roundtable,
predecessor to the Drug Forum

Building a National Framework for the Establishment of Regulatory Science for Drug Development—Workshop (February 2010)

In its 2007 report, *The Future of Drug Safety: Promoting and Protecting the Health of the Public*, the IOM identified the need for an improved science base for drug evaluation within FDA, including both internal resources and extramural funding for collaboration with academia. In that same year, the FDA Science Board, at the request of Congress, reported on the agency's need for an enhanced science base, including infrastructure development, multisector collaboration, and an expanded workforce capable of addressing the rapidly evolving science of drug discovery and development. In 2008, the Forum held a public workshop to explore the science of drug regulation, focusing on the gap between leading-edge technologies of drug development and FDA's capacity to adapt its process of regulatory evaluation to these technologies. Together, the results of these efforts suggest a widening gap between scientific developments in areas relevant to FDA's mission and its ability to address these innovations, as well as a lack of understanding among the public, policy makers, and the agency of what is required to fill this gap. To address these concerns, the Forum convened a public workshop, held February 26, 2010, to examine the state of the science of drug regulation and consider approaches for enhancing the scientific basis of regulatory decision making. The workshop provided an opportunity to explore the concept of regulatory science, examine how it can be used to improve regulatory decision making, and consider alternative mechanisms and institutional frameworks for its development and application.

March 23–24
Forum Meeting #1

June 29–30
Forum Meeting #2

September 8–9
Forum Meeting #3

Multidrug-Resistant Tuberculosis (MDR TB)

The Forum's initiative on multidrug-resistant tuberculosis (MDR TB) includes a series of workshops that have gained international attention. The first workshops—held in the United States in 2008 and 2009—highlighted new data in conducting a realistic assessment of the magnitude of the problem and the gaps needed to address the rapid spread of drug-resistant TB. These meetings led to the development of workshops to take place in the four highest burden countries—South Africa, Russia, India, and China. The Forum collaborated with the National Institute of Allergy and Infectious Diseases (NIAID) of NIH to develop coordinated MDR TB-related research meetings in both Russia and South Africa, and that collaboration is continuing in 2011 as the Forum plans its next workshop in New Delhi, India. The NIH meetings focus on science and opportunities for scientific collaboration, while the IOM meetings address health care delivery, drug access, public health, and other policy issues.

The Emerging Threat of Drug-Resistant Tuberculosis in Southern Africa: Global and Local Challenges and Solutions—Workshop (March 2010)

The first meeting in the workshop series on MDR TB was held in Pretoria, South Africa on March 3–4, 2010. The Forum partnered with the Academy of Science of South Africa (ASSAf) to convene the two-day workshop, which brought together disease experts, community leaders, and policymakers to examine the state of MDR TB in the South Africa region, to learn from the experiences of the South African public health community in its fight against MDR TB, and to draw lessons regarding best practices and novel approaches that can be applied both within and beyond the region. The South Africa meeting focused on various aspects of MDR TB, including epidemiology, diagnostics and preventive therapies, treatment, transmission and infection control, pediatric MDR TB, and public policy issues.



The New Profile of Drug-Resistant Tuberculosis in Russia: A Global And Local Perspective—Workshop (May 2010)

The second meeting in the workshop series on MDR TB was held in Moscow, Russia on May 26–27, 2010. The workshop was mentioned as one of the first activities in the Statement of Intent under a Memorandum of Understanding between the Academies, NIH, and the Russian Academy of Sciences signed during President Obama’s visit to Moscow in 2009. The Forum partnered with the Russian Academy of Medical Sciences to convene the two-day workshop, which brought together disease experts, community leaders, and policymakers. FDA Commissioner Dr. Margaret Hamburg and Dr. Paul Farmer of Partners In Health delivered speeches and participated in session discussions. Like the South Africa meeting, the meeting focused on various aspects of MDR TB (including epidemiology, diagnostics and preventive therapies, treatment, transmission and infection control, pediatric TB, and public policy issues). The meeting objectives were to examine the state of MDR TB in Russia, to learn from the experiences of the Russian and international public health community in its fight against MDR TB, and to draw lessons regarding best practices and novel approaches that can be applied both within and beyond the region.



Looking Forward

Forum Activities in 2011

Forum Meetings

The Forum plans to meet in March, June, and October 2011 to identify and discuss key problems and strategies in the discovery, development, and translation of drugs. In addition, the Forum is organizing a number of public meetings, described below.

Advancing Regulatory Science for Medical Countermeasure Development— An Institute of Medicine Workshop (March 2011)

The Drug Forum will again collaborate with the IOM's Forum on Medical and Public Health Preparedness for Catastrophic Events to organize a workshop that focuses on advancing regulatory science for MCM development. The workshop will examine ways to advance regulatory science for MCM development for chemical, biological, radiological/nuclear, and emerging infectious disease threats and identify scientific opportunities to improve, simplify, or speed MCM development and improve the predictability and success rate of candidate MCMs.

The Global Threat of Multidrug-Resistant Tuberculosis (India and China)

In April 2011, the Forum will hold an MDR TB workshop in New Delhi, India. The workshop, which represents a continuation of the Forum's international workshop series, will bring together local and global experts, as well as government leaders, to continue the dialogue on overcoming the rising threat of MDR TB. The Forum also plans to travel to China to hold its fourth workshop in the series in late 2011 or early 2012.





Members from left to right: Les Benet, Steven Galson, Annalisa Jenkins, and Jack Keene



Janet Woodcock

Envisioning a Transformed U.S. Clinical Trial Enterprise

In 2011, the Forum will continue its attention to how clinical trials can be improved to enhance feedback to a learning health care system, leading to a major workshop to be held in fall 2011. 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 by convening stakeholders to describe the core components of, and to set the agenda for, a transformed clinical trial enterprise. Through discussions and papers submitted to the meeting, the workshop will consider strategies for improving the organization and design of clinical trials across different settings and clinical areas. An additional meeting addressing approaches for enhanced public engagement in the clinical trial enterprise is also planned for 2011.

November 30
Discussion Series: A Conversation
with Tony Fauci

2008

February 20-21
Discussion Series: Comparative
Effectiveness (Forum Meeting #9)

April 21
Discussion Series:
Science at FDA: Challenges
and Opportunities

June 23-24
Symposium: Diseases and Individualized
Therapies (Forum Meeting #10)

June 23
Workshop: Breakthrough
Business Models: Drug
Development for Rare
and Neglected Diseases

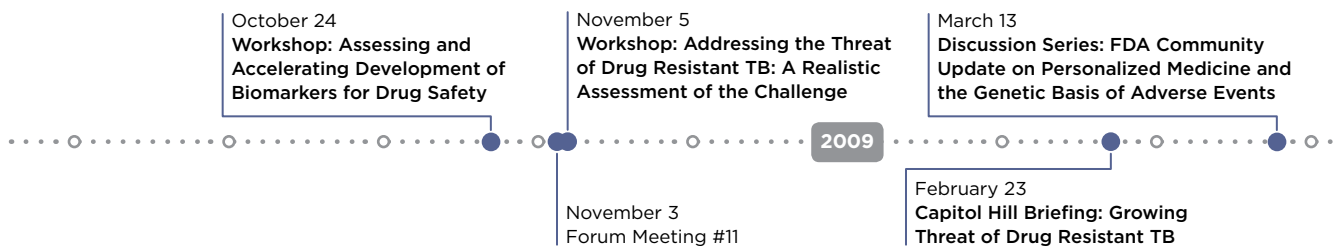
Forum Initiatives

Addressing the Approach to Drug Development: Problems and Opportunities

Recent years have seen both extraordinary opportunity and complex challenges in pharmaceutical innovation. Advances in genomic science, systems biology, and cell-based technologies have led to exciting new avenues of biomedical research and drug discovery and have given us glimpses of the “personalized” future of medicine. Yet despite these advances, the pathway from basic science to new therapeutics faces challenges on many fronts. The scientific challenges in finding novel drug targets are profound, and the translational divide results in only a small fraction of investigational new drugs reaching FDA approval and the patients who need them. The Forum has explored these issues from many perspectives—emerging technology platforms, regulatory efficiency, intellectual property concerns, the potential for precompetitive collaboration, and innovative business models that address the “valley of death”—and in the process has served to generate new ideas, inform policy and legislation, and provide a critical sounding board for FDA, NIH, and industry.

Promoting and Enhancing the Scientific Basis for the Regulation of Drugs

Over the past several years, the Forum has focused its attention on the scientific basis for the regulation of drugs. The Forum held a series of meetings on drug safety over the course of 2009, addressing topics of personalized medicine and the genetic basis of adverse events and advances in post-market drug safety since the passage of the Food and Drug Administration Amendments Act of 2007 (FDAAA), as well as Commissioner Peggy Hamburg’s vision for FDA. In February 2010, the Forum held a workshop that examined the state of the science of drug regulation and considered approaches for enhancing the scientific basis of regulatory decision making. Also, since 2007, the Forum has hosted public meetings designed to engage congressional staff and the broader policy community on current topics in drug development.



Transforming Research and Fostering Collaborative Research

The Forum recognizes that the critical link between bench and bedside in developing new diagnostics and therapeutic approaches is clinical research. The Forum tackled the issue head-on by establishing an initiative to examine the state of clinical trials in the United States, identify areas of strength and weakness in our current clinical trial enterprise, and consider transformative strategies for enhancing the ways in which clinical research is organized and conducted. The first workshop, held in October 2009, considered case studies in four disease areas to derive lessons that can be applied throughout clinical research. The Forum held two discussion meetings in 2010 to address issues of management of conflict of interest in biomedical research and regulatory and administrative impediments to the efficient and effective conduct of clinical trials. Meetings for 2011 are under development that will address moving toward greater public engagement in and understanding of the clinical trial enterprise, and envisioning a framework for a transformed national clinical trial enterprise. Also in 2010, the Forum convened two working groups in connection with its clinical trials initiative to address major themes and problems afflicting the U.S. clinical trial enterprise: one working group considered issues pertaining to the regulation and infrastructure of clinical trials in the United States, and the other looked at ways to enhance public engagement in the clinical research enterprise.



NIH's Francis Collins with HHS Secretary Kathleen Sebelius

April 27
**Workshop: Streamlining
Clinical Trial and Material
Transfer Negotiations**

April 27-28
Forum Meeting #12

September 2
**Discussion Series: FDA
Community Update on
Post-Market Drug Safety**

July 10
**Symposium: Drug Regulation with
FDA Commissioner, Peggy Hamburg**
(Forum Meeting #13)

October 15-16
Forum Meeting #14

October 7-8
**Workshop: Transforming Clinical
Research in the US**

Developing Drugs for Rare and Neglected Diseases and Addressing Urgent Global Health Problems

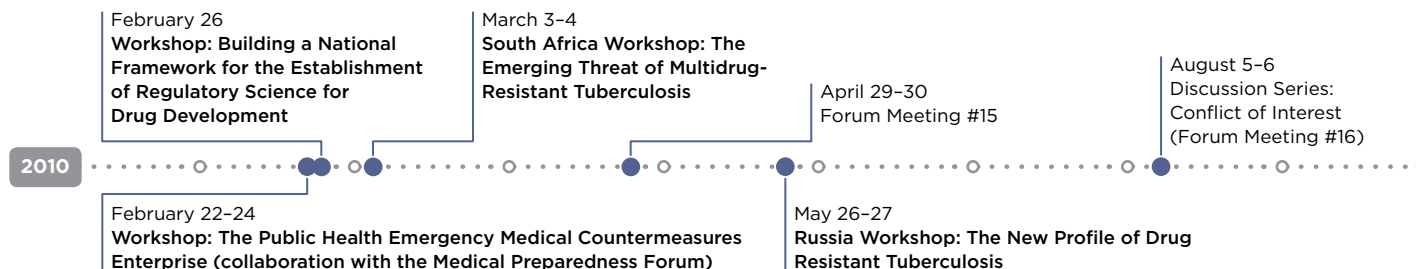
The Forum is sponsoring a series of workshops on the global problem of MDR TB. The Forum held a foundational workshop in Washington, DC in November 2008, for which it commissioned a paper from Partners In Health. Additional workshops are under way in the four countries with the highest MDR TB burden—South Africa (held March 2010), Russia (held May 2010), India (to be held April 2011) and China (anticipated late 2011 or early 2012). Also in 2011, the Forum will convene a focused initiative addressing the global drug supply chain for quality-assured second-line drugs for tuberculosis.



Paul Farmer, Partners In Health

Promoting Public Understanding of Drug Development

Successful introduction of new therapeutic entities requires testing in an informed and motivated public. The Forum has spent concerted effort to understand what limits public participation and how to enhance more widespread acceptance of the importance of advancing therapeutic development through public participation in the drug development process. Forum meetings held in the spring and fall of 2010 addressed these issues. The Forum plans to continue to work with multiple stakeholders to improve public understanding of and participation in the drug development process.



Reports Released in 2010



The Public Health Emergency Medical Countermeasures Enterprise—Workshop Summary

Released: April 8, 2010

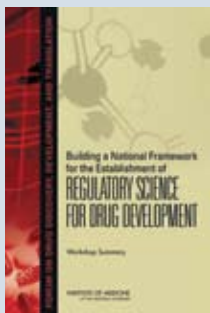
At the request of the Secretary of the U.S. Department of Health and Human Services and the Assistant Secretary for Preparedness and Response, the IOM held a workshop February 22–24, 2010, to address challenges facing the Public Health Emergency Medical Countermeasures Enterprise. Workshop participants discussed federal policies and procedures affecting the research, development, and approval of medical countermeasures and explored opportunities to improve the process and protect Americans' safety and health. The Forum on Medical and Public Health Preparedness for Catastrophic Events and the Forum on Drug Discovery, Development, and Translation collaborated on this workshop and summary. This document summarizes the workshop.



Transforming Clinical Research in the United States: Challenges and Opportunities—Workshop Summary

Released: August 2, 2010

The IOM held a public workshop October 7–8, 2009, to evaluate the state of clinical research in the United States, and to identify strategies for improving clinical trials' efficiency and effectiveness. Clinical trial experts discussed their successes, failures, and challenges in conducting clinical research. This document summarizes the workshop.



Building a National Framework for the Establishment of Regulatory Science for Drug Development—Workshop Summary

Released: October 8, 2010

The IOM held a public workshop on February 26, 2010, to examine the state of regulatory science and to consider approaches for enhancing it. The workshop provided an opportunity for stakeholders to clarify and explore the concept of regulatory science, examine how it can be used to improve regulatory decision making, consider its funding needs, and contemplate alternative mechanisms and institutional frameworks for its development and application. This document summarizes the workshop.

2011

October 29

Discussion Series: Administrative and Regulatory Inefficiencies
in Clinical Trials (Forum Meeting #17)

Forum Members

(as of December 31, 2010)

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.

Gail Cassell, (Co-Chair)

Harvard Medical School
Department of Global
Health & Infectious Disease
Research Institute

Jeffrey Drazen, (Co-Chair)

New England Journal
of Medicine

Barbara Alving

National Center for Research
Resources

Leslie Benet

University of California, San
Francisco

Ann Bonham

Association of American Medical
Colleges

Linda Brady

National Institute of Mental Health

Robert Califf

Duke University Medical Center

Scott Campbell

Foundation for the NIH

Thomas Caskey

University of Texas Health Science
Center at Houston

Peter Corr

Celtic Therapeutics, LLLP

James Doroshow

National Cancer Institute

Gary Filerman

Atlas Research

Garret FitzGerald

University of Pennsylvania
School of Medicine

Elaine Gallin

Doris Duke Charitable Foundation

Steven Galson

Amgen Inc.

Harry Greenberg

Stanford University School
of Medicine

Stephen Groft

NIH Office of Rare Disease Research

Peter Honig

AstraZeneca

Annalisa Jenkins

Bristol-Myers Squibb

Michael Katz

March of Dimes Foundation

Jack Keene

Duke University Medical Center

Ronald Krall

University of Pennsylvania

Freda Lewis-Hall

Pfizer, Inc.

William Matthew

National Institute
of Neurological Disorders
and Stroke

Mark McClellan

Brookings Institution

Carol Mimura

University of California,
Berkeley

John Orloff

Novartis Pharmaceuticals
Corporation

Amy Patterson

NIH Office of the Director

Janet Shoemaker

American Society for Microbiology

Ellen Sigal

Friends of Cancer Research

Ellen Strahlman

GlaxoSmithKline

Nancy Sung

Burroughs Wellcome Fund

Jorge Tavel

National Institute of Allergy and
Infectious Diseases

Janet Tobias

Ikana Media

Joanne Waldstreicher

Johnson & Johnson

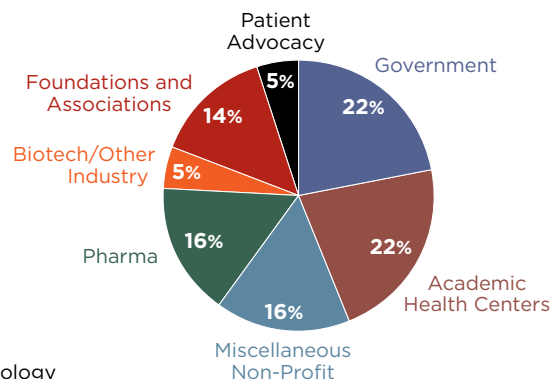
Janet Woodcock

FDA Center for Drug Evaluation
and Research

Raymond Woosley

The Critical Path Institute

Membership



Forum Sponsorship

(as of December 31, 2010)

Financial support for the Forum is derived from private foundations, government agencies, industry sponsors, and nonprofit associations.

Private Foundations

Burroughs Wellcome Fund
Doris Duke Charitable Foundation

Other Nonprofit Organizations

American Diabetes Association
American Society for Microbiology
Association of American Medical Colleges
Critical Path Institute
Friends of Cancer Research

Government Sponsors

Food and Drug Administration
National Cancer Institute (National Institutes of Health)
National Center for Research Resources (National Institutes of Health)
National Institute of Allergy and Infectious Diseases (National Institutes of Health)
National Institute of Mental Health (National Institutes of Health)
National Institute of Neurological Disorders and Stroke (National Institutes of Health)
Office of Rare Disease Research (National Institutes of Health)

Industry Sponsors

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Celtic Therapeutics, LLLP
Eli Lilly and Company
GlaxoSmithKline
Johnson & Johnson
Novartis
Pfizer, Inc.

About the Forum on Drug Discovery, Development, and Translation

The Forum on Drug Discovery, Development, and Translation was created in 2005 by the IOM's Board on Health Sciences Policy to provide an opportunity for stakeholders to meet and discuss issues of mutual interest in a neutral setting. The Forum offers a unique platform for dialogue and collaboration among its membership, which 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. To supplement the perspectives and expertise of its members, the Forum also holds public workshops to engage a wide range of experts, members of the public, and the policy community in discussing areas of concern in the science and policy of drug development. The Forum brings together thought leaders and stakeholders from private sector sponsors of biomedical and clinical research, federal agencies sponsoring and regulating biomedical and clinical research, the academic community, and consumers, and in doing so serves to educate the policy community about issues where science and policy intersect. Proceedings and speaker presentations are disseminated to the public through published summaries and the Forum website. For more information about the Forum on Drug Discovery, Development, and Translation, please visit our web site at: www.iom.edu/drug.

About the Institute of Medicine

The Institute of Medicine was established in 1970 by the National Academy of Sciences to secure the services of eminent members of appropriate professions in the examination of policy matters pertaining to the health of the public. The Institute acts under the responsibility assigned to the National Academy of Sciences by its congressional charter to serve as an advisor to the federal government and, upon its own initiative, to identify issues needing attention in the areas of medical care, research, and education.

Forum Staff

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Senior Program Assistant

Board on Health Sciences Policy

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Director



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The Institute of Medicine serves as adviser to the nation to improve health.

Established in 1970 under the charter of the National Academy of Sciences, the Institute of Medicine provides independent, objective, evidence-based advice to policymakers, health professionals, the private sector, and the public.

The mission of the Institute of Medicine embraces the health of people everywhere.