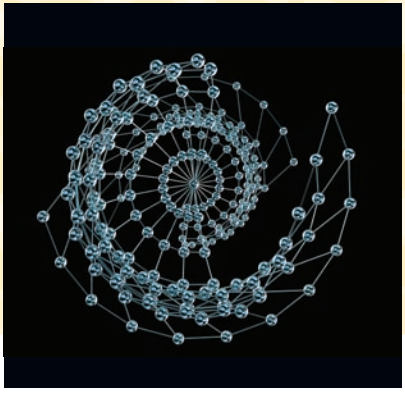
HEALTH AND MEDICINE DIVISION
BOARD ON HEALTH SCIENCES POLICY

# Forum on Drug Discovery, Development, and Translation

## 2015 Annual Report



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# Message from the Co-Chairs

**Russ Altman and Steven Galson**

The Forum on Drug Discovery, Development, and Translation of the National Academies of Sciences, Engineering, and Medicine (the Academies) was created in 2005 by the Academies' Board on Health Sciences Policy to foster communication, collaboration, and action in a neutral setting on issues of mutual interest relating to drug discovery, development, and translation. The Forum brings attention and visibility to important issues; 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 membership includes leaders from the Food and Drug Administration and the National Institutes of Health, the pharmaceutical and biotech industries, academia, foundations, and patient and disease advocacy organizations. The group is self-governing, with Forum members convening several times each year to identify and prioritize the topics they wish to address.

The Forum recognizes that although breakthroughs in biomedical research have led to an increased understanding of human disease, the translation of these discoveries into therapies for patients has not kept pace with medical need. The pharmaceutical innovation enterprise faces continued and mounting pressures, strained from all sides by increasing costs, suboptimal productivity, regulatory and economic uncertainties, and accelerating complexity. As diseases become increasingly complex and our knowledge about them becomes more comprehensive, the time is ripe for an increased investment in innovation and commitment to effective collaborative models and partnerships. When patients, industry, federal agencies, academia, and funders come together, their efforts can create results that would be impossible alone.

The Forum views challenges as opportunities and has used its convening to address a variety of extraordinarily complex issues. In 2015, the Forum provided a focused and neutral venue for stakeholders to identify and characterize the needs and priorities in the drug discovery and development "ecosystem" and to encourage meaningful information sharing and collaboration across sectors. The Forum contributed to broad conversations on drug development research and policy, including convening a workshop to explore a forward-looking agenda for bolstering the field of innovative regulatory science. The Forum membership also continued its focused effort to address challenges in the drug discovery and development process by facilitating an action-oriented collaborative that identified rate-limiting steps in the drug development enterprise and facilitated dialogue for potential process improvement efforts in the biomedical innovation ecosystem.

In 2016, a broad and deep policy conversation continues about how we can advance biomedical product innovation nationally and globally. Congressional discussions and federal agency initiatives have begun to address some key issues, including advancing the development of precision medicine, championing treatments for rare diseases, building a workforce prepared to address arising challenges in biomedical research, and facilitating clinical data sharing. The Executive Branch has launched the Cancer "Moonshot" initiative—a coordinated effort to enhance cooperation among researchers, regulators, and patients to accelerate the development of new cancer therapies. Informed deliberation is still necessary, however, to make progress and advance our system for developing promising discoveries into therapies for patients. The Forum will continue to convene its innovative action collaborative to establish a vocabulary and identify key bottlenecks in the biomedical innovation ecosystem. The Forum will also convene a workshop exploring approaches to maximize the utility of large genomic data banks to facilitate more productive drug discovery. Through these and other working group discussions and workshops, solicited and original qualitative research and collaborative writing, and broad outreach, the Forum will serve as a hub and a catalyst for new ideas and directions.

As a neutral convening venue for stakeholders and collaborators, the Forum provides a unique setting in which complex issues of health science policy can be tackled collegially, and in which partnerships may be formed and nurtured. We look forward to another groundbreaking and productive year for the Forum in 2016.

Russ Altman
Co-Chair

Steven Galson
Co-Chair

# Reflecting Back

**Forum Activities in 2015**

## Forum Meetings

The Forum membership met three times in 2015. Discussions at these meetings focused on diverse topics relating to the Forum's priorities, including mapping the biomedical innovation ecosystem; overcoming challenges in biomedical innovation; strengthening the regulatory science ecosystem; communicating uncertainty in the assessment of benefits and risks; strategies for responsible sharing of clinical trial data; and policy updates relevant to drug discovery, development, and translation. In addition, the Forum convened public workshops and collaborative activities, described below.

## Workshops:

### Financial Incentives to Support Unmet Medical Needs for Nervous System Disorders—Workshop (January 2015)

The global burden of nervous system disorders is projected to significantly increase over time. Although there have been recent international initiatives to better understand the human brain in order to develop new therapeutics, several large pharmaceutical companies have decreased investment or even withdrawn from their neuroscience research programs. The perceived high risk and low probability of success has made the neuroscience sector less attractive than other therapeutic areas for research and development (R&D), despite the large market potential. As a result, patients are often left with few, if any, options for treatment; thus, there is a need to consider policy options to increase private-sector investment in R&D for nervous system disorders. The Forum collaborated with the Forum on Neuroscience and Nervous System Disorders to convene this public workshop, which explored opportunities to foster private-sector innovation by supporting new investments directed toward the development of novel therapeutics to meet unmet needs for nervous system disorders.


(Left to right) Guest speaker Sally Rockey (NIH) with Lana Skirboll, Lynn Hudson, Andy Dahlem, Jack Keene, and Richard A. Moscicki at the Forum's March 2015 meeting.

## Enabling Rapid Response and Sustained Capability with Medical Countermeasures to Mitigate Risk of Emerging Infectious Diseases—Workshop (March 2015)

Global attention to recent large-scale outbreaks and their public health and medical consequences has made clear that the current medical countermeasure (MCM) response system is not well adapted to rapidly respond to a large number of diverse threats through adequate development and production of vaccines, therapeutics, diagnostic tools, and other non-pharmaceutical interventions. The Forum, in collaboration with the Forum on Medical and Public Health Preparedness for Catastrophic Events, co-convened a public workshop that examined how to better enable rapid and nimble private-sector engagement in the discovery, development, and translation of MCMs. This workshop explored what policies, guidance, and resources exist to guide decision making within the government and how the business and operational models employed by the private sector are affected by

policies and guidance and available resources set forth by the U.S. government. The discussions at this workshop were designed to identify and discuss what is needed to ensure that the private sector can respond in a rapid, nimble manner to ensure the availability of MCMs. The workshop also explored advances made by the Public Health Emergency Medical Countermeasures Enterprise (PHEMCE) to improve MCM development and translation.

### Priorities to Advance the Field of Regulatory Science: An Update on Progress and a Forward-Looking Agenda—Workshop (October 2015)

The Food and Drug Administration (FDA) today has a broad range of responsibilities, regulating approximately 25 percent of the U.S. economy. The agency has assumed an increasingly complex and international reach. In the face of rapid advances in medicine and biomedical science, FDA faces pressure to keep pace with new technologies and develop the expertise necessary to regulate those technologies as they emerge. The Forum, in collaboration with the Burroughs Wellcome Fund, convened a public workshop that discussed issues related to the development of the discipline of innovative regulatory science, focusing on infrastructure, systems, and workforce. The workshop featured invited presentations and discussions that explored current regulatory science priorities and strategies in federal, academic, and private-sector settings; considered the current state of regulatory science as a discipline; explored the core components of a robust discipline of innovative regulatory science; considered gaps and key opportunities to address needs to support the discipline of innovative regulatory science; and examined needs and barriers to collaboration among, across, and within the public and private sectors.

## Action Collaboratives:

### Mapping and Connecting the Biomedical Innovation Ecosystem—Action Collaborative

The biomedical innovation ecosystem is a dynamic network of activity. Standardizing and bringing clarity to this complex process could help to set a common vocabulary and allow more fluid dialogue among ecosystem participants to encourage further innovation. It could also facilitate ongoing discussion


*Priorities to Advance the Field of Regulatory Science* panelists (left to right) Stephen Ostroff, Alastair Wood, Martin Philbert, John Wagner, Brian Strom, and Darrell Abernethy.

**2007**

**March 12**
**Symposium: The Future of Drug Safety: Challenges for FDA**

**April 23–24**
**Workshop: Emerging Safety Science, FDA (Forum Meeting #7)**

**September 14**
**Discussion Series: From Patient Needs to New Drug Therapies**

**October 15–16**
**Forum Meeting #8**


(Left to right) FDA Commissioner Robert Califf, Forum Director Anne Claiborne, and Forum Co-Chairs Russ Altman and Steven Galson at the June 2015 meeting of the Forum.

to help frame, map, and synergize activities across the biomedical innovation ecosystem. Defining key terms, such as translational science and regulatory science, and locating complex activities within the biomedical innovation ecosystem landscape might help to articulate problem areas and provide opportunities to learn from local environments where the system is efficient and well-integrated with other areas. The goal of this collaborative is to enable discussion and to identify rate-limiting steps in order to facilitate process improvement efforts. In 2015, collaborative participants adapted two process maps for development of small molecules and biologics to identify inputs, bottlenecks, and needs. They also convened several meetings to discuss bottlenecks with key stakeholders and thought leaders, including Forum members. Collaborative participants are now in the process of authoring a Perspectives paper summarizing and developing a

**2008**

**November 30**
**Discussion Series: A Conversation with Tony Fauci**

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

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

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

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

plan for how these process maps could be used to help inform Forum priority setting. In 2016, a priority-setting tool developed from the Mapping initiative will be piloted and improved, and Forum meetings will provide opportunities for information sharing about key bottlenecks and science policy priorities in the biomedical innovation ecosystem.

### Disruptive Innovation and the Transformation of the Drug Development and Translational Science Enterprise—Action Collaborative

Many argue that the current paradigm for drug discovery and development requires disruptive innovation to break out of a crisis in R&D productivity. Evidence suggests that industries are almost always disrupted from the outside by new technologies they were slow to embrace, new business models they wrongly dismissed, or policy changes they thought they could keep at bay. The pharmaceutical industry offers many opportunities for disruption in each of these areas. The Forum convened an action collaborative that set out to identify and highlight potentially breakthrough ideas and visionary approaches to the "drug development and translational science enterprise of the future." The effort addressed new technologies (e.g., biosensors, apps and telemetry, synthetic biology, or new delivery technologies); new business models (e.g., crowdsourcing platforms, drug repurposing, virtual companies, or clinical trials); and policy issues (e.g., pricing/reimbursement, patent law, or data transparency). The effort included a data-gathering phase and review phase involving the Forum membership, and will include the preparation of a Perspectives paper.

**October 24**
**Workshop: Assessing and Accelerating Development of Biomarkers for Drug Safety**

**November 3**
**Forum Meeting #11**

**November 5**
**Workshop: Addressing the Threat of Drug-Resistant Tuberculosis: A Realistic Assessment of the Challenge**


*Priorities to Advance the Field of Regulatory Science* panelists (left to right) Stephen Ostroff, FDA Commissioner Robert Califf, Martin Landray, and Susan Ward.

## Clinical Trial Site Standards Harmonization—Action Collaborative

Since sponsoring a workshop series on issues relating to the U.S. national clinical trials enterprise from 2009 to 2011, the Forum continues to devote time and attention to issues around clinical trials. This action collaborative is an ad hoc convening activity under the auspices of the Forum, which provides a venue for joint and collaborative activities among participants to advance development of standards or a system to improve clinical trial performance through accreditation of clinical trial sites. Participants, who are drawn from multiple sectors and disciplines, are preparing a Perspectives paper that will summarize their perspectives on a process for standards development and on the establishment of a mechanism to facilitate coordination of an experimental approach to align existing standards and improve clinical trial site

**2009**

**February 23**
**Capitol Hill Briefing: Growing Threat of Drug-Resistant Tuberculosis**

**March 13**
**Discussion Series: FDA Community Update on Personalized Medicine and the Genetic Basis of Adverse Events**

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

**April 27–28**
**Forum Meeting #12**

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

standards based on continuous data collection. Collaborative participants have also undertaken a second phase of the collaborative activity, which includes the collection, analysis, and assessment of how clinical trial site standards currently in use by key stakeholders could be harmonized.

### Sharing Clinical Trial Data—Action Collaborative

Sharing clinical trial data can facilitate more efficient and effective development of better medicines, diagnostics, and procedures for the ultimate benefit of patients. At the same time, sharing data presents risks, burdens, and challenges that need to be addressed by a broad set of stakeholders. These opportunities and challenges were laid out in the Institute of Medicine (IOM) report, *Sharing Clinical Trial Data: Maximizing Benefits, Minimizing Risk*. The report calls on stakeholders to foster a culture of sharing and offers a blueprint for action within and across sectors. Four Academies Forums, including the this forum, provided momentum and a framework for initiating the IOM consensus study that produced the report, and are working together again to form a platform that could support coordination and collaboration among stakeholders engaged in data-sharing initiatives through convening and other activities. In 2015, collaborative participants launched the first workstream from this Data Sharing action collaborative, focusing on *Building an IT and Technical Infrastructure*, which is a collaboration with Harvard's Multi Regional Trial Center (MRCT) to convene stakeholders with relevant technical, legal, and content expertise to define a framework for the key issues in building and sustaining a global technical infrastructure.

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

**October 15–16**
**Forum Meeting #14**

**2010**

**October 7–8**
**Workshop: Transforming Clinical Research in the United States**

**February 22–24**
**Workshop: The Public Health Emergency Medical Countermeasures Enterprise (in collaboration with the Medical Preparedness Forum)**

**February 26**
**Workshop: Building a National Framework for the Establishment of Regulatory Science for Drug Development**

# Looking Forward

**Forum Activities in 2016**

### Forum Meetings

The Forum membership will meet in March, July, and October 2016 to continue its discussions of key problems and strategies in the discovery, development, and translation of drugs. Forum workshop planning committees, working groups, and action collaboratives will convene to discuss and act on priority areas identified by them, including the following activities:

## Action Collaboratives:

### Disruptive Innovation and the Transformation of the Drug Development and Translational Science Enterprise—Action Collaborative

The Forum may convene one or more workshops in 2016 to further explore issues identified through this effort, including defining a drug development paradigm for the future, considering the impact of new technologies on drug development, and opportunities for innovation at the academia–industry interface.

### Mapping and Connecting the Biomedical Innovation Ecosystem—Action Collaborative

In 2016, a priority-setting tool developed from the Mapping initiative will be piloted and improved, and Forum meetings will provide opportunities for information sharing about key bottlenecks and science policy priorities in the biomedical innovation ecosystem.

### Clinical Trial Site Standards Harmonization—Action Collaborative

Collaborative participants have undertaken a second phase of the collaborative activity, which includes the collection, analysis, and assessment of how clinical trial site standards

March 3–4
South Africa Workshop: The Emerging Threat of Multidrug-Resistant Tuberculosis

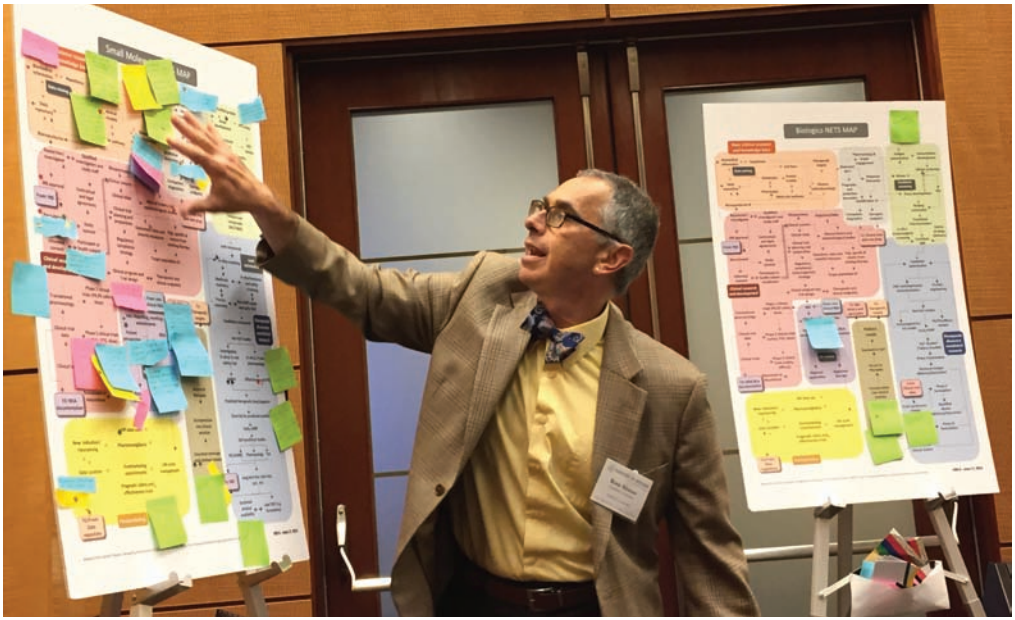
April 29–30
Forum Meeting #15

May 26–27
Russia Workshop: The New Profile of Drug-Resistant Tuberculosis

August 5–6
Discussion Series: Conflict of Interest (Forum Meeting #16)

October 29
Discussion Series: Administrative and Regulatory Inefficiencies in Clinical Trials (Forum Meeting #17)

currently in use by key stakeholders could be harmonized. In 2016, this analytic work will continue, Collaborative participants will convene to discuss a way forward, and Forum members will be engaged in opportunities for further action.



Russ Altman discusses the *Mapping and Connecting the Biomedical Innovation Ecosystem* flowchart during the June 2015 meeting of the Forum.

**2011**

**March 28**
**Forum Meeting #18**

**April 18–19**
**India Workshop: Facing the Reality of Multidrug-Resistant Tuberculosis**

**March 29–30**
**Workshop: Advancing Regulatory Science for Medical Countermeasure Development (in collaboration with the Medical Preparedness Forum)**

**June 27–28**
**Workshop: Public Engagement and Clinical Trials: New Models and Disruptive Technologies**

**June 28–29**
**Forum Meeting #19**

**July 12**
**South Africa Workshop Summary Report Release:** *The Emerging Threat of Multidrug-Resistant Tuberculosis: Global and Local Challenges and Solutions*

### Sharing Clinical Trial Data—Action Collaborative

The *Building an IT and Technical Infrastructure* working group convened on February 3, 2016, at the Academies in Washington, DC, and is expected to release its suggested way forward at a meeting at the Wellcome Trust in London in March 2016. Also being launched in 2016 under the auspices of the Data Sharing Action Collaborative is a workstream addressing *Data Sharing Practices and Standards in the Advocacy Communities*, which will convene nonprofit funders of research, focusing on disease advocacy and patient-focused organizations to develop standards and/or policies for fostering, promoting, or requiring data sharing by grantees.

## Workshop:

### Deriving Drug Discovery Value from Large-Scale Genetic Bioresources—Workshop (March 2016)

With the number of new drug approvals by the FDA remaining fairly constant during the past 60 years along with rising development costs, many pharmaceutical companies have examined innovative strategies to revitalize and create efficiencies in their drug development processes. One approach has involved the adoption of genetically guided strategies to reduce attrition rates and increase the odds of success. A recent analysis of approved medicines indicated that drugs supported by genetic evidence for the prescribed indication could have up to double the clinical success rate of those drugs that lack such genetic evidence. Several large cohort studies have incorporated or begun to incorporate genetic data collection as part of the study design. With the large volumes of genetic and phenotypic data that are planned to be collected, these

efforts could provide a valuable trove of information for identifying and validating potential targets, elucidating underlying disease and mechanistic biology, and developing biomarker assays and targeted therapies. Partnerships can foster development of innovative, precompetitive business models to more fully leverage genomic data. Questions remain about how large cohort studies could be designed, the types of data that should be collected, and which business models could engage stakeholders most effectively. The Forum, in collaboration with the Roundtable on Translating Genomic-Based Research for Health, will convene a workshop that will examine and discuss how large-scale genetic data could be used to improve the likelihood of bringing effective and targeted therapies to patients. The workshop will assess the current landscape of genomic-enabled drug discovery and development activities; examine how to enable partnerships and develop better business models; and consider gaps and best practices in how data from populations could be collected with the goal of improving the drug discovery process.

**June 4–5**
**Workshop: Maximizing the Goals of the Cures Acceleration Network to Accelerate the Development of New Drugs and Diagnostics**

**June 5**
**Forum Meeting #22**

**July 31–August 1**
**Workshop: Developing and Strengthening the Global Supply Chain for Second-Line Drugs for Multidrug-Resistant Tuberculosis**

**October 4–5**
**Workshop: Sharing Clinical Research Data (in collaboration with the Neuroscience Forum, National Cancer Policy Forum, and Genomics Roundtable)**

**October 23–24**
**Forum Meeting #23**

**November 26–27**
**Workshop: Large Simple Trials and Knowledge Generation in a Learning Health System (in collaboration with the Roundtable on Value & Science-Driven Health Care)**

# Forum Themes and Priorities

*The Forum addresses key problems in the discovery, development, and translation of drugs, covering the full translational continuum from basic discovery to the approval and adoption of new therapies into clinical practice. As an overarching and cross-cutting theme, the Forum fosters innovative efforts to identify and highlight potentially breakthrough ideas and visionary approaches to the* ***"drug development and translational science enterprise of the future."*** *The Forum has also identified four core components of translational science across this continuum that serve as thematic pillars to frame the Forum's focus areas and activities: (1) Innovation and the Drug Development Enterprise; (2) Science Across the Drug Development Lifecycle (Basic, Translational, and Regulatory Sciences); (3) Clinical Trials and Clinical Product Development; and (4) Infrastructure and Workforce for Drug Discovery, Development, and Translation.*

## Innovation and the Drug Development Enterprise

Despite exciting scientific advances, the pathway from basic science to new therapeutics faces challenges on many fronts. 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. There is also increasing recognition of the need for new models and methods for drug development and translational science, and "precompetitive collaborations" and other partnerships, including public–private partnerships, are proliferating. The Forum offers a venue to discuss effective collaboration in the drug discovery and development enterprise and also hosts discussions that could help chart a course through the turbulent forces of disruptive innovation in the drug discovery and development "ecosystem."

**December 19**
**Action Collaborative Meeting #1: Developing a National Accreditation System to Improve Clinical Trial Performance**

**2013**

**January 15**
**Workshop Summary Report Release:** *Developing and Strengthening the Global Supply Chain for Second-Line Drugs for Multidrug-Resistant Tuberculosis*

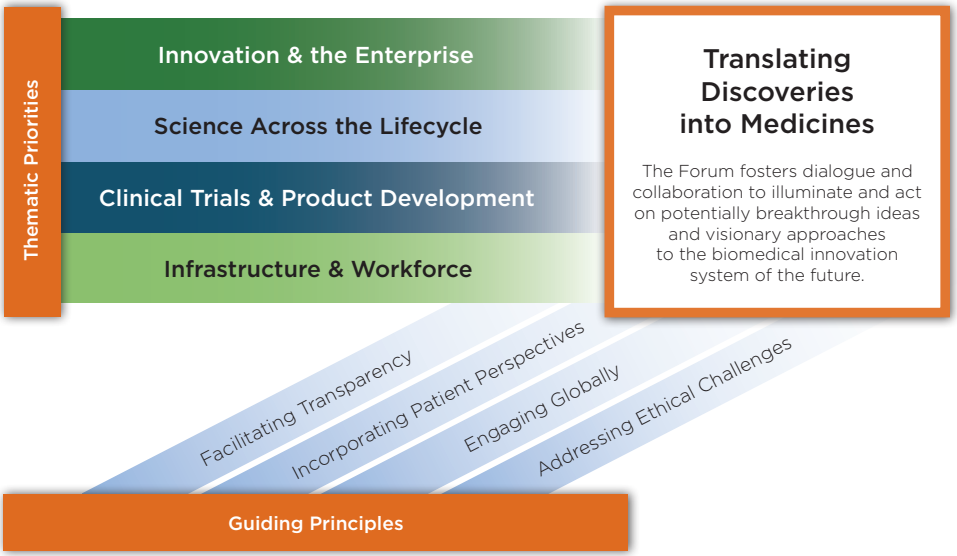
**January 16–18**
**China Workshop: The Global Crisis of Drug-Resistant Tuberculosis and Leadership of the BRICS Countries**

**February 12**
**Forum Meeting #24**

**February 13–14**
**Workshop: International Regulatory Harmonization Amid Globalization of Biomedical Research and Medical Product Development**

**June 3**
**Forum Meeting #25**

## Substance—What We Aim For




### Science Across the Drug Development Lifecycle

Key gaps remain in our knowledge about science, technology, and methods needed to support drug discovery and development. Recent rapid advances in innovative drug development science present opportunity for revolutionary developments of new scientific techniques, therapeutic products, and applications. The Forum provides a venue to focus ongoing attention and visibility to these important drug development needs and facilitates exploration of new approaches across the drug development lifecycle. The Forum has held workshops that have contributed to the defining and establishment of regulatory science and have helped inform aspects of drug regulatory evaluation.

**August 21**
**Action Collaborative Meeting #2: Developing a National Accreditation System to Improve Clinical Trial Performance**

**February 12**
**Workshop: Characterizing and Communicating Uncertainty in the Assessment of Benefits and Risks of Pharmaceutical Products (Day 1)**

**May 12**
**Workshop: Characterizing and Communicating Uncertainty in the Assessment of Benefits and Risks of Pharmaceutical Products (Day 2)**

**2014**

**October 28–29**
**Forum Meeting #26**

**March 3–4**
**Forum Meeting #27**

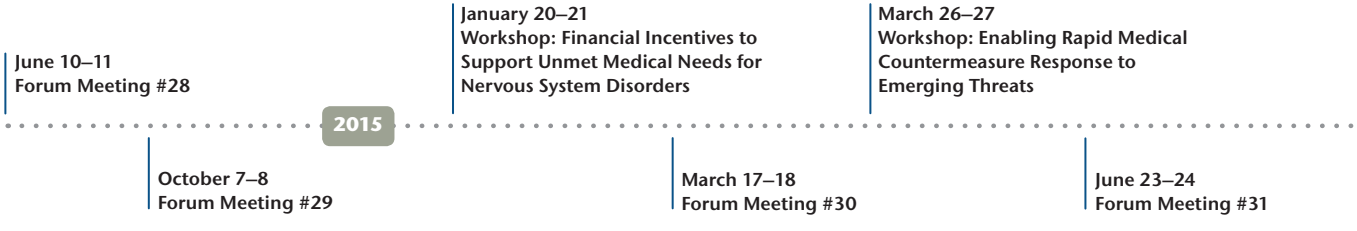
**March 11**
**Action Collaborative Meeting #3: Developing a National Accreditation System to Improve Clinical Trial Performance**

## Clinical Trials and Clinical Product Development

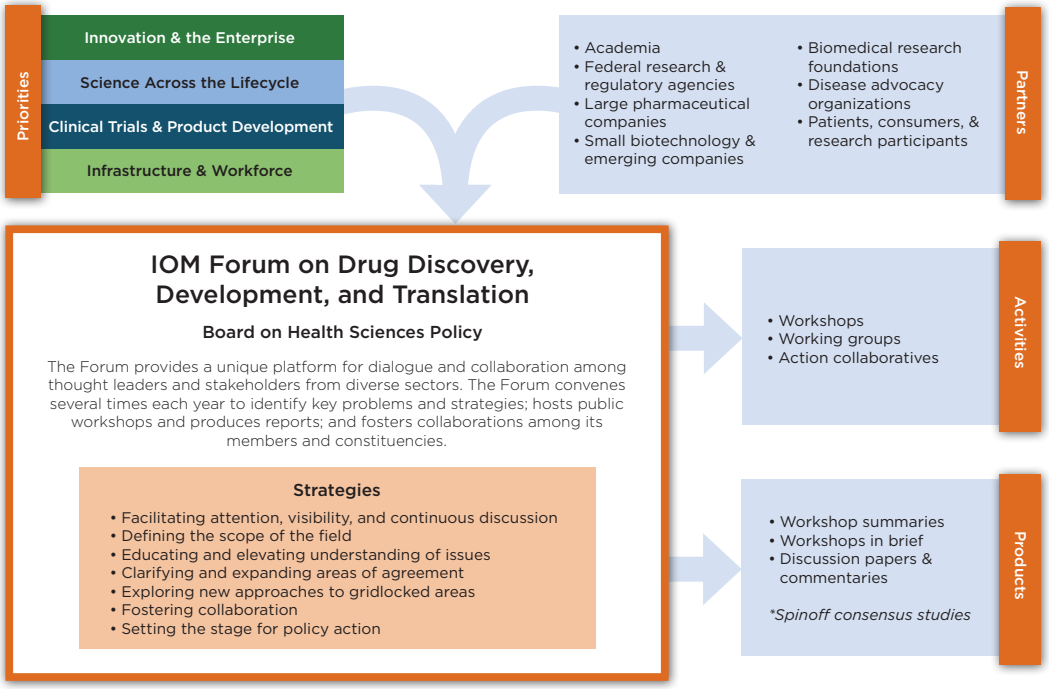
Clinical research is the critical link between bench and bedside in developing new therapeutics. Significant infrastructural, cultural, and regulatory impediments challenge efforts to integrate clinical trials into the health care delivery system. Collaborative, cross-sector approaches can help articulate and address these key challenges and foster systemic responses. The Forum has convened a multiyear 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 trials are organized and conducted. In addition to sponsoring multiple symposia and workshops, under this initiative, the Forum is fostering innovative, collaborative efforts to facilitate needed change in areas such as improvement of clinical trial site performance.

## Infrastructure and Workforce for Drug Discovery, Development, and Translation

Considerable opportunities remain for enhancement and improvement of the infrastructure that supports the drug development enterprise. That infrastructure, which includes the organizational structure, framework, systems, and resources that facilitate the conduct of biomedical science for drug development, faces significant challenges. The science of drug discovery and development, and its translation into clinical practice, is cross-cutting and multidisciplinary. Career paths can be opaque or lack incentives such as recognition, career advancement, or financial security. The Forum has considered workforce needs as foundational to the advancement of drug discovery, development, and translation. It has convened workshops examining these issues, including consideration of strategies for developing a discipline of innovative regulatory science through the development of a robust workforce. The Forum will also host an initiative that will address needs for a workforce across the translational science lifecycle.



## Process—How We Do It

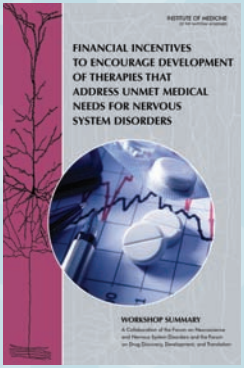



**October 20**
**Workshop: Priorities to Advance the Field of Regulatory Science**

**2016**

**October 21**
**Forum Meeting #32**

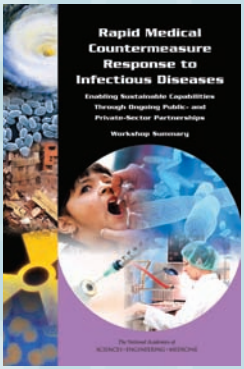
# Reports Released in 2015




**Financial Incentives to Encourage Development of Therapies That Address Unmet Medical Needs for Nervous System Disorders—Workshop Summary** (collaboration with the Forum on Neuroscience and Nervous System Disorders)

*Released: July 6, 2015*

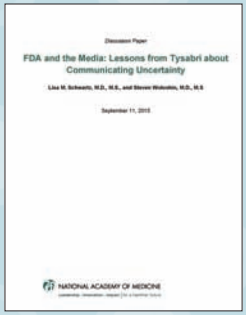
**The Forum on Neuroscience and Nervous System Disorders, in collaboration with the Forum on Drug Discovery, Development, and Translation, convened a workshop on January 20 and 21, 2015, to explore policy changes that might increase private-sector investment in research and development innovation that fills unmet medical needs for central nervous system (CNS) disorders. Workshop participants strategized about how to incentivize companies to fortify their CNS drug development programs, shrinking obstacles that currently deter ventures. Representatives from academia, government agencies, patient groups, and industry gathered to share information and viewpoints and to brainstorm about budget-neutral policy changes that could help widen the pipeline toward drugs that address unmet needs for CNS disorders. This report summarizes the presentations and discussions of the workshop.**




**Rapid Medical Countermeasure Response to Infectious Diseases: Enabling Sustainable Capabilities Through Ongoing Public- and Private-Sector Partnerships—Workshop Summary** (collaboration with the Forum on Medical and Public Health Preparedness for Catastrophic Events)

*Released: October 12, 2015*

**Emerging infectious disease threats that may not have available treatments or vaccines can directly affect the security of the world's health because these diseases also know no boundaries and will easily cross borders. Sustaining public and private investment in the development of MCMs before an emerging infectious disease becomes a public health emergency in the United States has been extremely challenging. Interest and momentum peak during a crisis and wane between events, and there is little interest in disease threats outside the United States until they impact people stateside. On March 26 and 27, 2015, the Forum on Medical and Public Health Preparedness for Catastrophic Events, in collaboration with the Forum on Drug Discovery, Development, and Translation, convened a workshop in Washington, DC, to discuss how to achieve rapid and nimble MCM capability for new and emerging threats. Public- and private-sector stakeholders examined recent efforts to prepare for and respond to outbreaks of Ebola Virus Disease, pandemic influenza, and coronaviruses from policy, budget, and operational standpoints. Participants discussed the need for rapid access to MCM to ensure national security and considered strategies and business models that could enhance stakeholder interest and investment in sustainable response capabilities. This report summarizes the presentations and discussions from this workshop.**




### FDA and the Media: Lessons from Tysabri about Communicating Uncertainty—Discussion Paper (By Lisa Schwartz and Steven Woloshin)

*Released: September 11, 2015*

**In light of a workshop held by the Forum on Drug Discovery, Development, and Translation in February and May 2014, "Characterizing and Communicating Uncertainty in the Assessment of Benefits and Risks of Pharmaceutical Products," which explored systematic and structured approaches to the characterization and communication of uncertainty, a discussion paper was developed by Lisa Schwartz and Steven Woloshin to further examine a case study presented at the workshop on the multiple sclerosis drug Tysabri.**

**Despite a rigorous approval process, there is always uncertainty about newly approved drugs: Will the benefits observed in initial approval studies hold up over time? Will side effects emerge? Uncertainty is amplified when drugs receive accelerated approval based on preliminary evidence or surrogate outcomes. Communicating uncertainty about new drugs is an important challenge facing FDA but opportunities exist to create realistic expectations and highlight the inherent uncertainty associated with accelerated approval. In this paper we examine how uncertainty was communicated by major media and FDA in the case of Tysabri, a multiple sclerosis drug, in order to draw lessons on how to improve the communication of uncertainty about new drugs.**

# Forum Sponsorship

**(as of December 31, 2015)**

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
FasterCures
Friends of Cancer Research
*New England Journal of Medicine*

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# Forum Members

**(as of December 31, 2015)**

Membership in the Forum includes a diverse range of stakeholders from multiple sectors, including government, the pharmaceutical and biotechnology industries, biomedical research funders and sponsors, academia, and patient groups.

**Russ Altman (Co-Chair)**
Stanford University

**Steven Galson (Co-Chair)**
Amgen Inc.

**Margaret Anderson**
FasterCures

**Hugh Auchincloss**
National Institute of Allergy and Infectious Diseases

**Christopher Austin**
National Center for Advancing Translational Sciences

**Ann Bonham**
Association of American Medical Colleges

**Linda Brady**
National Institute of Mental Health

**Gail Cassell**
Harvard Medical School Department of Social and Global Medicine (Visiting)

**Andrew Dahlem**
Eli Lilly & Co.

**James Doroshow**
National Cancer Institute

**Jeffrey Drazen**
*New England Journal of Medicine*

**Harry Greenberg**
Stanford University School of Medicine

**Lynn Hudson**
Critical Path Institute

**S. Claiborne (Clay) Johnston**
Dell Medical School at the University of Texas, Austin

**Jack Keene**
Duke University Medical Center

**Rusty Kelley**
Burroughs Wellcome Fund

**Ronald Krall**
University of Pittsburgh

**Freda Lewis-Hall**
Pfizer Inc.

**Briggs Morrison**
AstraZeneca

**Bernard Munos**
InnoThink Center for Research in Biomedical Innovation

**Elizabeth (Betsy) Myers**
Doris Duke Charitable Foundation

**Rajesh Ranganathan**
National Institute of Neurological Disorders and Stroke

**Robert Ratner**
American Diabetes Association

**Michael Rosenblatt**
Merck & Co., Inc.

**Michael Severino**
AbbVie

**Ellen Sigal**
Friends of Cancer Research

**Lana Skirboll**
Sanofi

**Murray Stewart**
GlaxoSmithKline

**Brian Strom**
Rutgers, the State University of New Jersey

**Janet Tobias**
Ikana Health

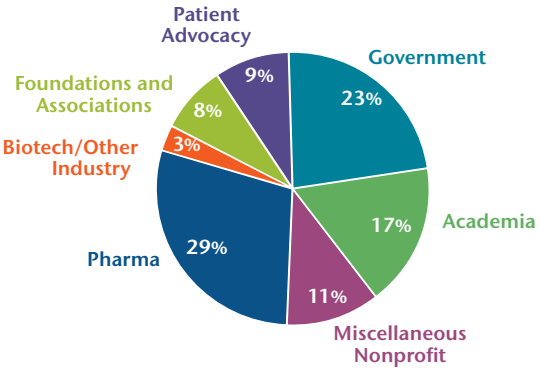
**John Wagner**
Takeda Pharmaceuticals

**Joanne Waldstreicher**
Johnson & Johnson

**Carrie Wolinetz**
NIH, Office of Science Policy

**Janet Woodcock**
FDA, Center for Drug Evaluation and Research

## Membership



## About the Forum on Drug Discovery, Development, and Translation

The Forum on Drug Discovery, Development, and Translation of the National Academies of Sciences, Engineering, and Medicine was created in 2005 by the Academies' Board on Health Sciences Policy to provide a unique platform for dialogue and collaboration among thought leaders and stakeholders in government, academia, industry, foundations, and patient advocacy with an interest in improving the system of drug discovery, development, and translation. The Forum brings together leaders from private-sector sponsors of biomedical and clinical research, federal agencies sponsoring and regulating biomedical and clinical research, the academic community, and patients, and in doing so serves to educate the policy community about issues where science and policy intersect.

The Forum convenes several times each year to identify and discuss key problems and strategies in the discovery, development, and translation of drugs. 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. The Forum also fosters collaborations among its members and constituencies. For more information about the Forum on Drug Discovery, Development, and Translation, please visit our website at www.hmd.nationalacademies.org/drug.

### Forum Staff

**Anne B. Claiborne, J.D., M.P.H.**
*Senior Program Officer and Forum Director*

**Christopher J. DeFeo, Ph.D.**
(until October 2015)
*Program Officer*

**Rebecca A. English, M.P.H.**
*Program Officer*

**Amanda Wagner Gee, M.S.**
(as of November 2015)
*Program Officer*

**Emily R. Busta, M.S.**
(until December 2015)
*Associate Program Officer*

**Morgan L. Boname, M.S.**
(as of December 2015)
*Associate Program Officer*

**Kathryn M. Howell**
(until November 2015)
*Senior Program Assistant*

**Olivia C. Yost, M.S.**
(as of December 2015)
*Senior Program Assistant*

### Board on Health Sciences Policy

**Andrew M. Pope, Ph.D.**
*Director*

## About the National Academies of Sciences, Engineering, and Medicine

The **National Academy of Sciences** was established in 1863 by an Act of Congress, signed by President Lincoln, as a private, nongovernmental institution to advise the nation on issues related to science and technology. Members are elected by their peers for outstanding contributions to research. Dr. Ralph J. Cicerone is president.

The **National Academy of Engineering** was established in 1964 under the charter of the National Academy of Sciences to bring the practices of engineering to advising the nation. Members are elected by their peers for extraordinary contributions to engineering. Dr. C. D. Mote, Jr., is president.

The **National Academy of Medicine** (formerly the Institute of Medicine) was established in 1970 under the charter of the National Academy of Sciences to advise the nation on medical and health issues. Members are elected by their peers for distinguished contributions to medicine and health. Dr. Victor J. Dzau is president.

The three Academies work together as the **National Academies of Sciences, Engineering, and Medicine** to provide independent, objective analysis and advice to the nation and conduct other activities to solve complex problems and inform public policy decisions. The Academies also encourage education and research, recognize outstanding contributions to knowledge, and increase public understanding in matters of science, engineering, and medicine.

Learn more about the National Academies of Sciences, Engineering, and Medicine at **www.national-academies.org**.

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The nation turns to the National Academies of Sciences, Engineering, and Medicine for independent, objective advice on issues that affect people's lives worldwide.
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