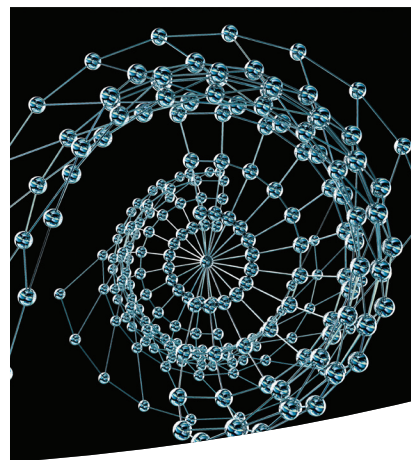


# Forum on Drug Discovery, Development, and Translation

**Board on Health Sciences Policy**



**2017 Annual Report**



*The National Academies of*  
**SCIENCES • ENGINEERING • MEDICINE**



## Message from the Co-Chairs

Russ Altman and Steven Galson

**The Forum provides a unique platform for dialogue and collaboration among thought leaders and stakeholders in government, academia, industry, foundations, and disease and patient advocacy with an interest in improving the system of drug discovery, development, and translation, educating the policy community about issues where biomedical science and policy intersect.**



Forum on  
**DRUG DISCOVERY,  
DEVELOPMENT,  
and TRANSLATION**

Despite breakthroughs in biomedical research that have led to an increased understanding of human disease, the translation of discoveries into therapies for patients has not kept pace with medical need. The biomedical innovation enterprise faces continued and mounting pressures, strained from all sides by increasing costs, suboptimal productivity, regulatory and economic uncertainties, and increasing clinical trial complexity. As our technical capabilities and understanding of diseases and medical conditions become more comprehensive, the time is ripe for 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 lead to results that would be impossible to achieve in isolation.

The Forum on Drug Discovery, Development, and Translation (Forum) of the National Academies of Sciences, Engineering, and Medicine (National Academies) was created in 2005 by the National Academies' Board on Health Sciences Policy to foster communication, collaboration, and action in a neutral setting on issues of mutual interest across the drug research and development (R&D) lifecycle. The Forum membership includes leaders from the U.S. Food and Drug Administration (FDA) and the National Institutes of Health (NIH), the biopharmaceutical industry, academia, consortia, foundations, journals, and patient focused and disease advocacy organizations. Through the Forum's activities, participants have been better able to bring attention and visibility to important issues, explore new approaches for resolving problem areas, share information and find common ground, and work together to develop ideas into concrete actions and new collaborations.

In 2017, the Forum contributed to broad conversations on important topics, including the launch of a three-part workshop series to explore a forward-looking agenda for generating and incorporating real-world evidence into medical product development and evaluation. The Forum also co-hosted the second workshop

in a two-part series on the role of genetics in clinical drug development, further underscoring the critical role of precision medicine across the drug R&D lifecycle. Among its membership, the Forum discussed emerging and pertinent issues and legislative and regulatory policy updates relevant to drug discovery, development, and translation.

In 2018, a broad and deep policy conversation continues regarding how to advance biopharmaceutical innovation nationally and globally. The executive and legislative branches of the federal government have continued to show bipartisan support for biomedical research, including ongoing funding for programs such as the BRAIN Initiative, the Precision Medicine Initiative, and the 21st Century Cures Act, as well as passage of the Food and Drug Administration Reauthorization Act, which includes the sixth reauthorization of the Prescription Drug User Fee Act. As a result, government funders and regulators of biomedical research such as FDA and the NIH stand to receive continued infusions of funding and support for relevant programmatic priorities.

The Forum serves as a hub and catalyst for nurturing new ideas and partnerships and is well positioned to contribute toward policy discussions to help promote the goals and sustain the momentum spurred by these and other initiatives as they progress in 2018 and beyond. Looking ahead, the Forum plans to focus on important issues such as advancing the science of patient input in medical product R&D, transforming clinical trials through digital health, addressing late-stage failures, and continuing activities to advance the use of real-world evidence in medical product development and evaluation, develop data sharing goals for nonprofit funders of clinical trials, and accelerate progress toward harmonization of clinical trial site standards.

We look forward to another groundbreaking and productive year for the Forum in 2018.



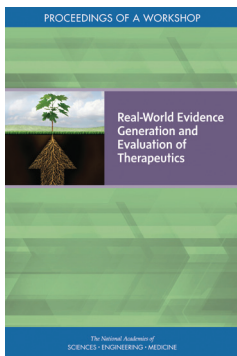
**Russ Altman**  
*Co-Chair*



**Steven Galson**  
*Co-Chair*

# Reflecting Back: Forum Activities in 2017

**Members of the Forum convene several times each year to identify, discuss, and act on key problems and strategies in the discovery, development, and translation of drugs.**



## **Forum Membership Meetings**

The Forum membership met three times in 2017. Discussions at these meetings focused on diverse topics related to the Forum's priorities, including the role of genetics in clinical drug development; improving the drug development process through examining late-stage failures; real-world evidence and medical product development; clinical trials and digital health; harmonizing clinical trial site standards; exploring the application of engineering principles to the clinical trials system; academic credit for data sharing; data sharing principles for nonprofit clinical trial funders; the science of patient input in medical product R&D; data science; tracking and communicating the Forum's impact; and other policy updates relevant to drug discovery, development, and translation. In addition, the Forum convened public workshops and collaborative activities, described below.

## **Workshops**

### **Real-World Evidence Generation and Evaluation of Therapeutics—A Workshop**

The traditional process for evaluating new therapeutics does not produce the evidence that patients, clinicians, and payers need for real-world decisions. The volume and complexity of information about individual patients are greatly increasing with the use of electronic records and personal devices. The potential effects on medical product development in the context of this wealth of real-world data are numerous and varied, ranging from the ability to determine both large-scale and patient-specific effects of treatments to assessing how therapeutics affect patients'

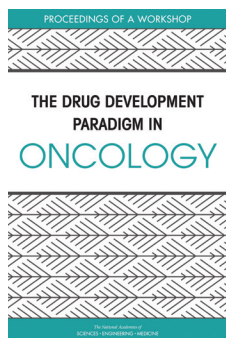




Forum members Clay Johnston and Joanne Waldstreicher look on during a presentation by National Library of Medicine (NLM) Director Patricia Brennan on data science at the NLM at the July 2017 Forum meeting.

lives through the measurement of lifestyle changes. However, mechanisms to facilitate the efficient use of real-world data to meet the decision-making needs of myriad stakeholders have not been established. This workshop, held on **October 19, 2016**, examined opportunities and challenges incorporating real-world evidence into the evaluation of medical products, including consideration of quality, relevance, and validity of real-world data sources; methodologies and best practices for the incorporation of real-world data throughout the medical product lifecycle; and the use of novel approaches such as Web-based or other digital technologies. The workshop proceedings, released on **February 15, 2017**, summarizes the presentations and discussions at the workshop.

**To supplement the perspectives and expertise of its members, the Forum holds public workshops to focus substantial public attention on critical areas of drug discovery and development. Proceedings of these workshops are disseminated to the public.**



### **The Drug Development Paradigm in Oncology—A Workshop**

Advances in cancer research have led to an improved understanding of the molecular mechanisms underpinning the development of cancer and how the immune system responds to cancer. This influx of research has led to an increasing number and variety of cancer therapies in the drug development pipeline. Compared with standard chemotherapies, these new cancer therapies may demonstrate evidence of benefit at an earlier stage of development. However, there is a concern that the traditional processes for cancer drug development, evaluation, and regulatory approval could impede or delay the use of these promising cancer treatments in clinical practice. This has led to a number of efforts—by patient advocates, the pharmaceutical industry, and FDA—to accelerate the development and evaluation of promising new cancer therapies, especially for cancers that currently lack effective treatments.

To explore this new landscape in cancer drug development, the National Cancer Policy Forum, in collaboration with the Forum, hosted a 1.5-day public workshop, The Drug Development Paradigm in Oncology, on **December 12–13, 2016**. The workshop convened cancer researchers, patient advocates, and representatives from industry, academia, and government to discuss challenges with traditional approaches to drug development, opportunities to improve the efficiency of drug development, and strategies to enhance the information available about a cancer therapy throughout its lifecycle in order to improve its use in clinical practice. The workshop proceedings, released on **July 24, 2017**, summarizes the presentations and discussions at the workshop.



### **Enabling Precision Medicine: The Role of Genetics in Clinical Drug Development—A Workshop**

Those involved in the drug development process face challenges of efficiency and overall sustainability due in part to high research costs, lengthy development timelines, and late-stage drug failures. Novel clinical trial designs that enroll participants based on their genetics represent a potentially disruptive change that could improve patient outcomes, reduce costs associated with drug development, and further realize the goals of precision medicine. On **March 8, 2017**, the Forum and the Roundtable on Genomics and Precision Health hosted the workshop

Enabling Precision Medicine: The Role of Genetics in Clinical Drug Development. This workshop examined successes, challenges, and possible best practices for effectively using genetic information in the design and implementation of clinical trials to support the development of precision medicines, including exploring the potential advantages and disadvantages of such trials across a variety of disease areas. Workshop participants were asked to take a general, high-level approach in their examination of these issues, to take a pulse of the successes that have been realized and the challenges that have been encountered, and to consider how these experiences might inform the advancement of precision medicine. The workshop proceedings, released on **July 10, 2017**, summarizes the presentations and discussions at the workshop.

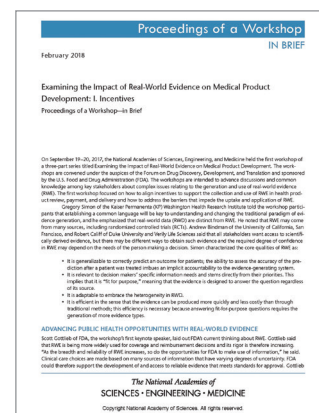
## Examining the Impact of Real-World Evidence on Medical Product Development—A Workshop Series • Workshop 1: Incentives

The Forum is convening a three-part workshop series, sponsored by FDA, examining how real-world evidence development and uptake can enhance medical product development and evaluation. The workshops will advance discussions and common knowledge about complex issues relating to the generation and utilization of real-world evidence, including fostering development and implementation of the science and technology of real-world evidence generation and utilization. The first workshop in the series was held on **September 19–20, 2017**, and focused on how to align incentives to support the collection and use of real-world evidence in medical product review, payment, and delivery, including incentives needed to address barriers impeding the uptake of real-world evidence, such as transparency. A workshop proceedings—in brief was released on **February 12, 2018**, summarizing the presentations and discussions at the workshop.

Workshop two will be a “town hall” style meeting with in-depth audience discussion and active participation to illuminate what types of data are appropriate for what specific purposes and to suggest practical approaches for data collection and evidence use by developing and working through example use cases. The third and final workshop will examine and suggest approaches for operationalizing the collection and use of real-world evidence.



Members of a panel session during the Enabling Precision Medicine workshop, co-convened with the National Academies' Roundtable on Genomics and Precision Health, participated in a moderated discussion with workshop attendees on the topic of integrating genetics into drug development for complex diseases.







## UPDATE ON OUR RECOMMENDATIONS

Gives us a better picture of our medicine and health



# Looking Forward: Forum Activities in 2018

## Forum Membership Meetings

The Forum membership will meet in March, July, and October 2018 to continue its discussions of key problems and strategies in the discovery, development, and translation of drugs. Forum workshop planning committees and action collaboratives will convene to discuss and act on priority areas identified by them, including the following workshops.

## Workshops

### **Examining the Impact of Real-World Evidence on Medical Product Development: A Workshop Series • Workshop 2: Practical Approaches and Workshop 3: Operationalizing**

The Forum is convening a three-part workshop series, sponsored by FDA, examining how real-world evidence development and uptake can enhance medical product development and evaluation. The workshops will advance discussions and common knowledge about complex issues relating to the generation and utilization of real-world evidence, including fostering development and the implementation of the science and technology of real-world evidence generation and utilization. Workshop One (**September 19–20, 2017**) focused on how to align incentives to support the collection and use of real-world evidence in medical product review, payment, and delivery, including incentives needed to address barriers impeding the uptake of real-world evidence, such as transparency. Workshop Two (**March 6–7, 2018**) will be a “town hall” style meeting with in-depth audience discussion and active participation to illuminate what types of data are appropriate for

**Building on priorities identified by Forum members during the course of Forum meetings, the Forum membership envisions future workshops and collaborative activities.**

Incoming Forum Director Carolyn Shore joins former Forum Director Anne Claiborne at the July 2017 meeting of the Forum.



**Workshops inform Forum meeting discussions and can spur the formation of action collaboratives or future workshops/workshop series.**

what specific purposes and to suggest practical approaches for data collection and evidence use by developing and working through example use cases. Workshop Three **(July 17–18, 2018)** will examine and suggest approaches for operationalizing the collection and use of real-world evidence.

**Advancing the Science of Patient Input in Medical Product R&D—  
Towards a Research Agenda: A Workshop**

The Forum will convene a 1-day workshop on **May 9, 2018**, focused on the science of patient input for pre-market medical product R&D (including pre-discovery, discovery, pre-clinical development, and clinical development), with consideration of downstream regulatory and post-market decision making. At this workshop, subject matter experts representing a range of disciplines will engage in presentations and discussions to:

- Examine the current state of the science, including successes and limitations of current efforts, for soliciting and incorporating patient input in pre-market R&D.
- Explore gaps in the knowledge base and other barriers that impede progress, including but not limited to:
  - parameters for soliciting and incorporating different types of patient input along the spectrum of medical product R&D;
  - availability, accessibility, and applicability of data sources for patient input;
  - tools and methodologies for collecting and analyzing patient input to produce scientifically rigorous, credible evidence; and
  - understanding of the ethical and value considerations for soliciting and incorporating patient input into medical product R&D decision making.
- Discuss a potential framework for and components of a research agenda for addressing gaps or barriers to realizing a science of patient input.

**Transforming Clinical Trials Through Digital Health:  
A Workshop (Q3/Q4 2018)**

Clinical trials are a cornerstone of medical product development—supporting the evaluation of efficacy and the identification of safety issues for new drugs and devices—and a necessary regulatory requirement for bringing novel therapies to market. This workshop will provide a venue to explore opportunities for a modern clinical trials enterprise capable of efficiently answering questions about medical products by bringing participants closer to the clinical trial through digital health tools. Since 2009, the Forum has been engaged in a focused effort, anchored by a multi-workshop series, to address challenges facing the U.S. clinical trials enterprise and to engage stakeholders in an ongoing discussion of potentially transformative strategies to improve the efficiency and effectiveness of clinical trials. This workshop will build on this work and examine opportunities for a modern clinical trials enterprise in light of digital health tools. Participants will highlight opportunities for systemic improvements in the clinical trials enterprise; consider novel platform designs and digital health tools to gain efficiency and enhance the interface between clinicians and participants in a clinical trial; and discuss digital platforms to facilitate recruitment and maintain diverse participation in clinical trials.



Forum member and former FDA Commissioner Robert Califf shares a lighthearted moment with current FDA Commissioner Scott Gottlieb during a Q&A session at the Forum's Real-World Evidence workshop in September 2017.

# Action Collaboratives<sup>1</sup>

**The Forum fosters action collaboratives to engage participants with similar interests and responsibilities in cooperative activities that provide in-depth analysis of high-priority issues and advance identified goals of the Forum and progress on recommendations highlighted in previous National Academies consensus study reports.**

## **Advancing the Science of Patient Input in Medical Product R&D**

There is growing momentum to incorporate patient input into medical product R&D and regulatory decision-making processes. Converting traditionally anecdotal patient input to rigorous, credible evidence for use by a broad range of stakeholders—including academic and clinical researchers, medical product developers, patient and disease advocacy groups, and regulatory decision makers—could better align medical product development and regulation with patient perspectives on disease experience, burden, management, and treatment. Although many efforts have been launched to advance a science of patient input, there is a critical need to identify gaps in the knowledge base and other barriers that are impeding progress and to develop a commonly held research agenda for addressing them. An action collaborative was launched in February 2017 to identify the areas of most critical need in the science of patient input that could be best advanced through cross-sector collaboration in the neutral venue of the National Academies. During the first phase of this project, completed in July 2017, Collaborative participants developed structured surveys to query experts and thought leaders on the state of the field and identify priority areas to be addressed by the project. Throughout the latter half of 2017, Collaborative participants aided in scoping and planning for the second phase of the Collaborative's work, a public, discussion-based workshop. In 2018, the second

<sup>1</sup> Each action collaborative is an ad hoc activity associated with the Forum at the National Academies. The work each produces does not necessarily represent the views of any one organization, the Forum, or the National Academies and is not subjected to the review procedures of, nor is it a report or product of, the National Academies.

phase of the Collaborative's work will take the form of a Forum-convened workshop, Advancing the Science of Patient Input in Medical Product R&D—Towards a Research Agenda. Discussions at this workshop will inform the project's anticipated third phase, involving strategic follow-up discussions and the development of a research framework for advancing the science of patient input.

## Clinical Trial Site Standards Harmonization

Since hosting 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. Collaborative participants, who are drawn from multiple sectors and disciplines, released a Discussion Paper in October 2017 that summarized their perspectives on a process for standards development and on the establishment of a mechanism to facilitate the coordination of an experimental approach to align existing standards and improve clinical trial site standards based on continuous data collection. In 2017, participants also continued work on a second phase of the Collaborative, including collection, analysis, assessment, and prioritization of clinical trial site standards that are currently in use by some research sponsors and how they could be harmonized.

In 2018, Collaborative participants will continue the second phase of their work, to include the review and refinement of a large set of clinical trial site standards that are currently in use and were identified during phase one of the project. The end-goal of this second phase is to suggest a set of core clinical trial site standards that are robust enough for launching the majority of phase II, III, and IV clinical trials and could potentially be considered for broad application by research sponsors.

## Associated NAM Perspective

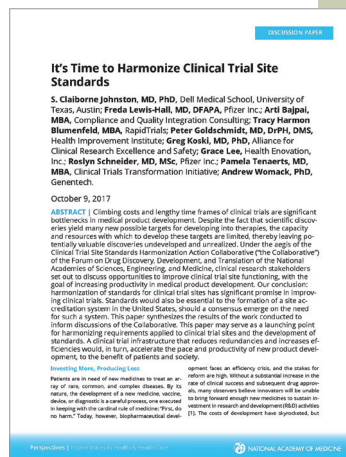
Climbing costs and lengthy time frames of clinical trials are significant bottlenecks in medical product development. Despite the fact that scientific discoveries yield many new possible targets for developing into therapies, the capacity and resources with which to develop these targets are limited, thereby leaving potentially valuable discoveries undeveloped and unrealized. Under the aegis of the Clinical Trial Site Standards Harmonization Action Collaborative associated with the Forum, clinical research stakeholders set out to discuss opportunities to improve clinical trial site functioning with the goal of increasing productivity in medical product development. The authors concluded that harmonization of standards for clinical trial sites would have significant promise in improving clinical trials. Standards would also be essential to the formation of a site accreditation system in the United States should a consensus emerge on the need for such a system. This paper synthesizes the results of the work conducted to inform discussions of the Collaborative and may serve as a launching point for harmonizing requirements applied to clinical trial sites and the development of standards. A clinical trial infrastructure that reduces redundancies and increases efficiencies would, in turn, accelerate the pace and productivity of new product development to the benefit of patients and society.

## It's Time to Harmonize Clinical Trial Site Standards

by S. Claiborne Johnston, Freda Lewis-Hall, Arti Bajpai, Tracy Harmon Blumenfeld, Peter Goldschmidt, Greg Koski, Grace Lee, Roslyn Schneider, Pamela Tenaerts, and Andrew Womack

October 9, 2017

*Disclaimer: The views expressed in this paper are those of the authors and not necessarily of the authors' organizations, the National Academy of Medicine (NAM), or the National Academies of Sciences, Engineering, and Medicine (the National Academies). The paper is intended to help inform and stimulate discussion. It is not a report of the NAM or the National Academies. Copyright by the National Academy of Sciences. All rights reserved.*



**Collaboratives are participant-driven, action-oriented activities that foster collaboration and information sharing among Forum members and external thought leaders and stakeholders.**

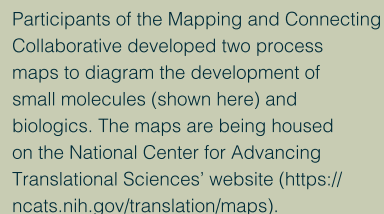
### **Improving the Drug Development Process Through Examining Late-Stage Failures**

The development of new drugs is inherently complex and costly, with sources documenting up to \$2.6 billion and more than 9 years in investment to bring a new treatment to market. The high cost is driven, at least in part, by the failure rate for new compounds entering late-stage development, but also encompasses opportunity costs due to time and resources not spent on developing an alternate treatment candidate, as well as investment in clinical trial infrastructure. Most importantly, these failures have an impact on patients—those who participate in the clinical trials associated with the testing of a failed drug candidate and those who will not benefit from having access to treatments that could have been developed instead. Given these considerations, late-stage development warrants further attention to understand why and how late-stage failures occur and to explore ways to improve the rate of success. The goal of this Collaborative is to examine the contributing factors to late-stage failures and develop a set of key considerations for stakeholders to improve the probability of success in late-stage product development. In 2018, Collaborative participants will help conduct structured, qualitative interviews with subject matter experts; analyze and synthesize responses from these interviews and consider information compiled from a literature survey; and plan a meeting to discuss and inform the key considerations.

### **Mapping and Connecting the Biomedical Innovation Ecosystem**

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 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.





## Associated Peer-Reviewed Articles



### A Dynamic Map For Learning, Communicating, Navigating, and Improving Therapeutic Development

by John Wagner, Andrew M. Dahlem, Lynn D. Hudson, Sharon F. Terry, Russ B. Altman, C. Taylor Gilliland, Christopher DeFeo, and Christopher P. Austin

*Nature Reviews Drug Discovery*

December 22, 2017

### Application of a Dynamic Map for Learning, Communicating, Navigating, and Improving Therapeutic Development

by John Wagner, Andrew M. Dahlem, Lynn D. Hudson, Sharon F. Terry, Russ B. Altman, C. Taylor Gilliland, Christopher DeFeo, and Christopher P. Austin

*Clinical and Translational Science*

December 22, 2017

This Collaborative fostered discussion to identify rate-limiting steps in order to facilitate process improvement efforts.

In 2017, Collaborative participants further refined two process maps that diagram the development of small molecules and biologics, which are being digitally hosted by the National Center for Advancing Translational Sciences at NIH (see previous page). The process maps—coined the Drug Discovery, Development, and Deployment Maps (4DM)—served as the foundation for two complementary peer-reviewed journal articles co-authored by Collaborative participants to guide future dialogue and progress on this topic, released in December 2017 (see sidebar on left). These articles describe the Collaborative’s year-long effort to identify rate-limiting steps in the ecosystem—informed and augmented by a diverse group of experts external to the Collaborative—and to develop accurate and dynamic maps to diagram the web of small molecule and biologics discovery, development, regulation, and delivery. The utility and application of the process maps as customizable models and discussion tools for diverse stakeholders to explore the intricate and intertwined processes of drug development are also highlighted.

Although this Collaborative has sunset as a formal convening activity associated with the Forum as of December 2017, the Forum may continue to support the dissemination and use of the process maps and other derivative products or activities resulting from the Collaborative’s robust work on this topic.

## Sharing Clinical Trial Data

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 National Academies forums and roundtables, including the Forum, provided momentum and a framework for initiating the IOM consensus study that produced

the report and are working together again to provide a platform to support coordination and collaboration among stakeholders engaged in data sharing initiatives through convening and other activities.

The first workstream from this Collaborative is convening nonprofit funders of research—focusing on disease advocacy and patient-focused organizations—to develop goals for fostering, promoting, or requiring data sharing by grantees. This working group of the Collaborative met in November 2017 to solicit feedback from a broad range of nonprofit funders and refine its draft *Statement of Data Sharing Goals for Nonprofit Funders of Clinical Trials*, including discussion of associated risks and challenges of the goals and next steps for operationalizing them. The Collaborative also continued to co-host a workstream in 2017 on *Building an IT and Technical Infrastructure*, a collaboration with Harvard's Multi-Regional Clinical Trials 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 clinical trial data sharing platform.

In 2018, the Forum will continue to serve as a sounding board for global collaborations to advance data sharing, including the anticipated public launch of Vivli—a new independent data repository, cloud-based analytics platform and in-depth search engine that will house data from clinical trials conducted by researchers from various organizations and entities. The Forum will also continue to support the refinement, dissemination, and uptake of the *Statement of Data Sharing Goals for Nonprofit Funders* being developed under the Collaborative's namesake workstream.

# Forum Themes and Priorities

**The Forum aims to understand and improve the system of drug discovery, development, and translation in a rapidly changing scientific and regulatory environment in order to improve the public's health.**

*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. The overarching theme underpinning all of the Forum's activities, workshops, publications, and working groups is to understand and improve the system of drug discovery, development, and translation in a rapidly changing scientific and regulatory environment in order to improve the public's health. The Forum has also identified four core components across this translational continuum that serve as thematic pillars to frame the Forum's focus areas and activities: (1) Innovation and Reform of 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 Reform of the Drug Development Enterprise**

Despite exciting scientific advances, the pathway from basic science to new therapeutics faces challenges on many fronts. Innovative 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 to advance drug discovery, development, and translational science. To address these needs, "precompetitive collaborations" and other partnerships, including public-private partnerships, are proliferating. The Forum offers a venue to discuss

effective collaborative opportunities across the drug discovery and development enterprise and hosts discussions that could help chart a course through the turbulent forces of disruptive innovation in the drug discovery and development “enterprise.”

### **Science Across the Drug Development Lifecycle**

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

### **Clinical Trials and Clinical Product Development**

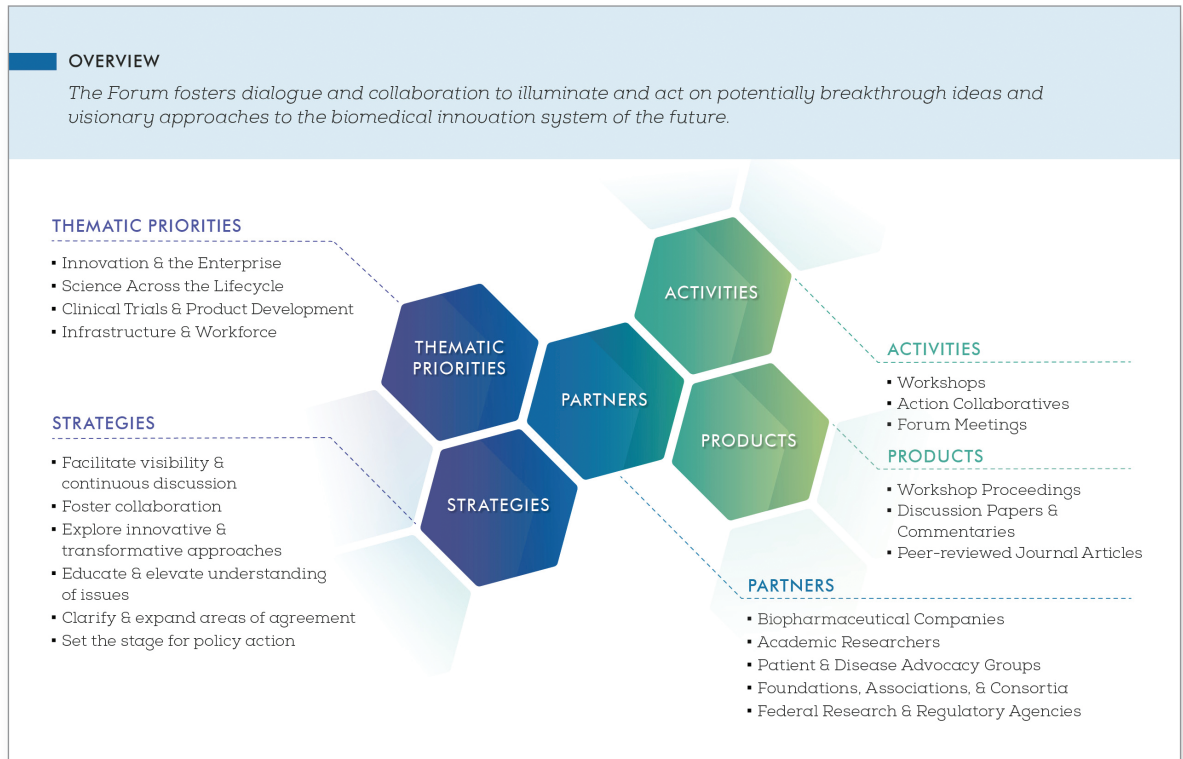
Clinical research is a critical link between bench and bedside in developing new therapeutics. Significant infrastructural, cultural, and regulatory impediments challenge efforts to integrate clinical trials into the health care delivery system. Collaborative, cross-sector approaches can help articulate and address these key challenges and foster systemic responses. In addition to sponsoring symposia and workshops to examine the state of clinical trials in the United States, the Forum fosters innovative, collaborative efforts to facilitate needed change in areas such as the improvement of clinical trial site performance and sharing of clinical trial data.

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

Considerable opportunities remain for the enhancement and improvement of the infrastructure that supports the drug discovery and development enterprise. That infrastructure, which includes the organizational structure, framework, systems, and resources that facilitate the conduct of biomedical science for drug development,

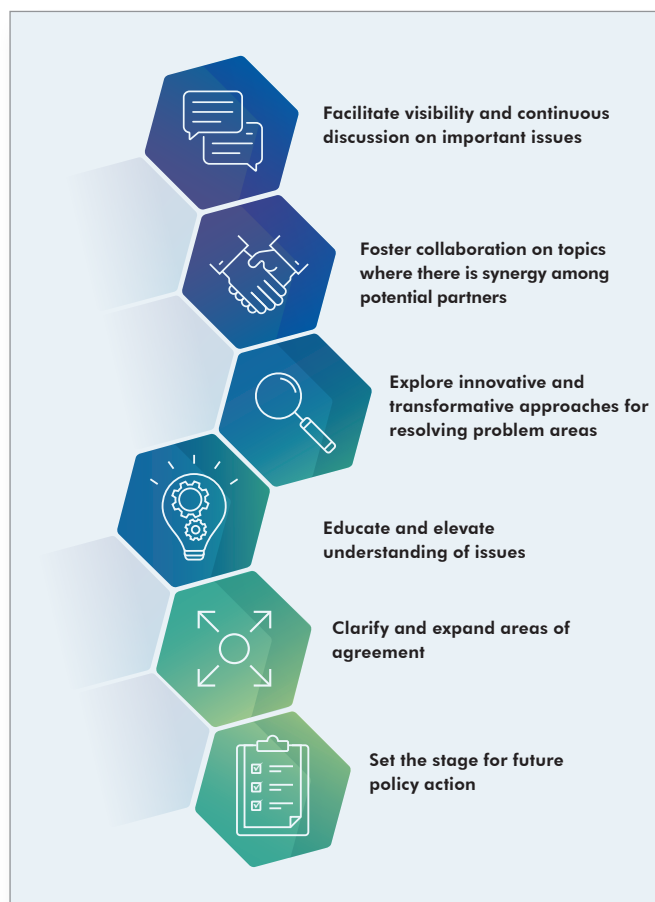


faces significant challenges. The science of drug discovery and development, and its translation into clinical practice, is cross-cutting and multidisciplinary. Career paths can be opaque or lack incentives such as recognition, career advancement, or financial security. The Forum has considered workforce 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.



### Strategies

Topics addressed by the Forum span a broad range of issues in drug discovery, development, policy, and practice. In providing a venue for independent, systematic discussions of these issues, the Forum employs the following strategies.



## SPONSORS

(through December 31, 2017)

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

### Government

Center for Drug Evaluation and Research, U.S. FDA  
Office of the Commissioner, U.S. FDA  
National Cancer Institute, NIH  
National Center for Advancing Translational Sciences, NIH  
National Institute of Allergy and Infectious Diseases, NIH  
National Institute of Mental Health, NIH  
National Institute of Neurological Disorders and Stroke, NIH  
Office of Science Policy, NIH

### Industry

AbbVie Inc.  
Amgen Inc.  
AstraZeneca  
Eli Lilly and Company  
GlaxoSmithKline  
Johnson & Johnson  
Merck & Co., Inc.  
Pfizer Inc.  
Sanofi  
Takeda Pharmaceuticals

### Private Foundations

Burroughs Wellcome Fund

### 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*

## MEMBERS

(through December 31, 2017)

Membership in the Forum includes a diverse range of stakeholders from multiple sectors, including government, biopharmaceutical industry, biomedical research funders and sponsors, academia, foundations, consortia, disease advocacy, and patient-focused groups.

### Russ Altman (*Co-Chair*)

Stanford University

### Steven Galson (*Co-Chair*)

Amgen Inc.

### Hugh Auchincloss

National Institute of Allergy and Infectious Diseases, NIH

### Christopher Austin

National Center for Advancing Translational Sciences, NIH

### Linda Brady

National Institute of Mental Health, NIH

### Robert Califf

Duke University and Verily Life Sciences

### Gail Cassell

American Society of Microbiology

### Andrew Dahlem

Eli Lilly and Company

### Tamara Darsow

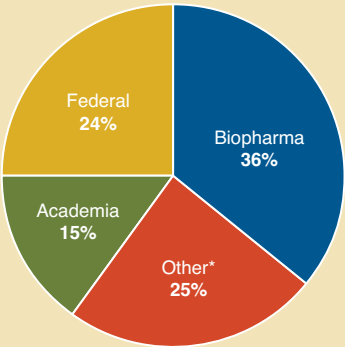
American Diabetes Association

Forum Sponsorship and Membership

- James Doroshow**  
National Cancer Institute, NIH
- Jeffrey Drazen**  
*New England Journal of Medicine*
- Julie Gerberding**  
Merck & Co., Inc.
- Jodie Gillon**  
Achillion
- Lynn Hudson**  
Critical Path Institute
- S. Claiborne Johnston**  
Dell Medical School, University of Texas, Austin
- Gregory Keenan**  
AstraZeneca
- Rusty Kelley**  
Burroughs Wellcome Fund
- Katharine Knobil**  
GlaxoSmithKline
- Freda Lewis-Hall**  
Pfizer Inc.
- Ross McKinney**  
Association of American Medical Colleges
- Bernard Munos**  
InnoThink Center for Research in Biomedical Innovation
- John Orloff**  
Alexion

- Michael Rosenblatt**  
Flagship Pioneering
- Michael Severino**  
AbbVie Inc.
- Rachel Sherman**  
Office of the Commissioner, U.S. FDA
- Ellen Sigal**  
Friends of Cancer Research
- Lana Skirboll**  
Sanofi
- Brian Strom**  
Rutgers, the State University of New Jersey
- Amir Tamiz**  
National Institute of Neurological Disorders and Stroke, NIH
- John Wagner**  
Takeda Pharmaceuticals
- Joanne Waldstreicher**  
Johnson & Johnson
- Carrie Wolinetz**  
Office of Science Policy, NIH
- Janet Woodcock**  
Center for Drug Evaluation and Research, U.S. FDA

Membership



\*Consortia, Foundations, Journals, Patient-Focused/ Disease Advocacy Organizations

# TIMELINE

**2002–2004** | Clinical Research Roundtable, predecessor to the Forum

**2005** | Forum on Drug Discovery, Development, and Translation founded | **March 23–24** Forum Meeting #1 | **June 29–30** Forum Meeting #2 | **September 8–9** Forum Meeting #3 | **November 3–4** Workshop: Adverse Drug Event Reporting: The Roles of Consumers and Health-Care Professionals

**2006** | **March 28–29** Forum Meeting #4 | **May 30–31** Workshop: Understanding the Benefits and Risks of Pharmaceuticals | **June 13** Workshop: Addressing the Barriers to Pediatric Drug Development | **June 27–28** Forum Meeting #5 | **October 24–25** Forum Meeting #6

**2007** | **March 12** Symposium: The Future of Drug Safety: Challenges for the FDA | **April 23–24** Workshop: Emerging Safety Science | **April 24** Forum Meeting #7 | **September 14** Discussion Series: From Patient Needs to New Drug Therapies: Can We Improve the Pathway? | **October 15–16** Forum Meeting #8 | **November 30** Discussion Series: A Conversation with Tony Fauci

**2008** | **February 20–21** Forum Meeting #9 | **February 20** Discussion Series: Comparative Effectiveness | **April 21** Discussion Series: Science at FDA: Challenges and

Opportunities | **June 23** Workshop: Breakthrough Business Models: Drug Development for Rare and Neglected Diseases and Individualized Therapies | **June 24** Forum Meeting #10 | **October 24** Workshop: Assessing and Accelerating Development of Biomarkers for Drug Safety | **November 2–3** Forum Meeting #11 | **November 5** Workshop: Addressing the Threat of Drug-Resistant Tuberculosis: A Realistic Assessment of the Challenge

**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 and Forum Meeting #13 | **September 2** Discussion Series: FDA Community Update: Improving the Science of Drug Safety | **October 7–8** Workshop: Transforming Clinical Research in the United States | **October 15–16** Forum Meeting #14

**2010** | **February 23–24** 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 | **March 3–4** Workshop: The Emerging Threat of Multidrug-Resistant Tuberculosis (Pretoria, South



Africa) | **April 29–30** Forum Meeting #15 | **May 26–27** Workshop: The New Profile of Drug-Resistant Tuberculosis (Moscow, Russia) | **August 5** Forum Meeting #16: Conflict of Interest | **October 29** Forum Meeting #17: Administrative and Regulatory Inefficiencies in Clinical Trials

**2011** | **March 28** Forum Meeting #18 | **March 29–30** Workshop: Advancing Regulatory Science for Medical Countermeasure Development (*in collaboration with the Medical Preparedness Forum*) | **April 18–19 and 21** Workshop: Facing the Reality of Multidrug-Resistant Tuberculosis: Challenges and Potential Solutions (New Delhi, India) | **June 27–28** Workshop: Public Engagement and Clinical Trials: New Models and Disruptive Technologies (*in collaboration with the Mount Sinai School of Medicine*) | **June 28–29** Forum Meeting #19 | **July 12** Workshop Summary Report Release: The Emerging Threat of Multidrug-Resistant Tuberculosis: Global and Local Challenges and Solutions (Durban, South Africa) | **September 20–21** Workshop: Strengthening a Workforce for Innovative Regulatory Science in Therapeutics Development | **October 4–5** Forum Meeting #20 | **November 7–8** Workshop: Envisioning a Transformed Clinical Trials Enterprise in the United States: Establishing an Agenda for 2020 | **November 15** Workshop Summary Report Release: The New Profile of Drug-Resistant Tuberculosis: A Global and Local Perspective (Moscow, Russia)

**2012** | **March 13–14** Forum Meeting #21 | **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 Leadership Consortium for a Value & Science-Driven Health System*)

**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** Workshop: The Global Crisis of Drug-Resistant Tuberculosis and Leadership of the BRICS Countries (Beijing, China) | **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 | **October 28–29** Forum Meeting #26

# TIMELINE

**2014 | February 12** Workshop: Characterizing and Communicating Uncertainty in the Assessment of Benefits and Risks of Pharmaceutical Products (Day 1) | **March 3–4** Forum Meeting #27 | **May 12** Workshop: Characterizing and Communicating Uncertainty in the Assessment of Benefits and Risks of Pharmaceutical Products (Day 2) | **June 10–11** Forum Meeting #28 | **October 7–8** Forum Meeting #29

**2015 | January 20–21** Workshop: Financial Incentives to Support Unmet Medical Needs for Nervous System Disorders (*in collaboration with the Neuroscience Forum*) | **March 17–18** Forum Meeting #30 | **March 26–27** Workshop: Rapid Medical Countermeasure Response to Infectious Diseases: Enabling Sustainable Capabilities Through Ongoing Public- and Private-Sector Partnerships (*in collaboration with the Medical Preparedness Forum*) | **June 23–24** Forum Meeting #31 | **October 20** Workshop: Advancing the Discipline of Regulatory Science for Medical Product Development: An Update on Progress and a Forward-Looking Agenda | **October 21** Forum Meeting #32

**2016 | March 22** Workshop: Deriving Drug Discovery Value from Large-Scale Genetic Bioresources (*in collaboration with the Genomics Roundtable*) | **March 23** Forum Meeting #33 | **July 19–20** Forum Meeting #34 | **October 18** Forum Meeting #35 | **October 19** Workshop: Real-World Evidence Generation and Evaluation of Therapeutics | **December 12–13** Workshop: The Drug Development Paradigm in Oncology (*in collaboration with the National Cancer Policy Forum*)

**2017 | March 8** Workshop: Enabling Precision Medicine: The Role of Genetics in Clinical Drug Development (*in collaboration with the Genomics Roundtable*) | **March 9** Forum Meeting #36 | **July 10–11** Forum Meeting #37 | **September 19–20** Workshop Series: Examining the Impact of Real-World Evidence on Medical Product Development—Workshop 1: Incentives | **October 24–25** Forum Meeting #38

## **FORUM STAFF**

### **Carolyn K. Shore, Ph.D.**

*Senior Program Officer and Forum Director  
(as of August 2017)*

### **Anne C. Claiborne, J.D., M.P.H.**

*Senior Program Officer and Forum Director  
(until August 2017)*

### **Rebecca A. English, M.P.H.**

*Program Officer*

### **Amanda Wagner Gee, M.S.**

*Program Officer*

### **Morgan L. Boname, M.S.**

*Associate Program Officer*

### **Melvin Joppy**

*Senior Program Assistant (as of October 2017)*

### **Michael Berrios**

*Senior Program Assistant (until August 2017)*

## **BOARD ON HEALTH SCIENCES POLICY**

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

*Director*

## ABOUT THE FORUM

The Forum on Drug Discovery, Development, and Translation (Forum) of the National Academies of Sciences, Engineering, and Medicine (National Academies) was created in 2005 by the National 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 <http://www.nationalacademies.org/DrugForum>.

## 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. Marcia McNutt 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 National 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.nationalacademies.org](http://www.nationalacademies.org).

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