



Forum on
Drug Discovery,
Development,
and Translation

Board on Health Sciences Policy



YEAR
REVIEW
2011–2020

The National Academies of
SCIENCES • ENGINEERING • MEDICINE



Over the coming decade, the forum will continue to serve as a hub and catalyst for nurturing new ideas and partnerships.



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

Message from the Co-Chairs

Robert Califf and Gregory Simon

Real progress in health and health care requires continuous innovation at every stage of the treatment lifecycle: discovery of new disease mechanisms and treatment targets, translation of those discoveries into effective and safe treatments, and delivery of those treatments to maximize public health benefit. Clinical trials remain a cornerstone of medical product development by providing the scientific evidence that proves or disproves concepts developed during earlier stages of development about the safety and efficacy of medical products and by informing clinical care. At the same time, the clinical research enterprise faces continued and mounting pressures, strained from all sides by rising costs, an evolving regulatory and economic landscape, increasing clinical trial complexity, difficulties in the recruitment and retention of research participants, and a clinical research workforce that is under tremendous stress. These challenges cannot be overcome in isolation and will require collaboration among patients, providers, academia, industry, federal agencies, payers, nonprofit organizations, and funders.

Consider the past—when health records were all on paper. Today, health systems serve millions of people and new technologies help improve our understanding of treatment and disease through analysis of information from electronic health records (EHRs) and other data sources. We have entered an exciting time in biomedical research when clinical research and health care are at a critical juncture and the biological, physical, and digital spheres are merging. These opportunities hold great promise for advancing our understanding of health maintenance and prevention, disease progression, and developing new therapies for patients.

The Forum on Drug Discovery, Development, and Translation (the forum) of the National Academies of Sciences, Engineering, and Medicine (the 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 National Institutes of Health (NIH), the U.S. Food and Drug Administration (FDA), the biopharmaceutical and digital technology industries, 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.

Over the past decade, the forum has supported a variety of activities that have engaged key stakeholders from across the biomedical research lifecycle. These activities have fostered discussion and collaboration toward a clinical trials enterprise that is more efficient, effective, patient-centered, and integrated into the health delivery system.

Looking ahead to the coming decade, more work is needed to spur biomedical innovation in a responsible and equitable manner and to ensure that research is adequately powered to answer practical questions about the safety and effectiveness of medical products. While we can point to important breakthroughs in understanding disease biology and the development of precision treatments, broader innovation is needed to address major causes of disability and premature mortality.

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 NIH Brain Research Through Advancing Innovative Neurotechnologies® (BRAIN) Initiative, the Precision Medicine Initiative, the 21st Century Cures Act, and a proposed new entity, the Advanced Research Projects Agency for Health (ARPA-H). Government funders and regulators of biomedical research, such as NIH and FDA, stand to receive continued infusions of funding and support for relevant programmatic priorities.

Over the coming decade, the forum will continue to serve as a hub and catalyst for nurturing new ideas and partnerships and offer a neutral space for stakeholders to advance critical policy discussions on biopharmaceutical innovation nationally and globally.



Robert Califf
Co-Chair

A handwritten signature in black ink that reads "Robert Califf".



Gregory Simon
Co-Chair

A handwritten signature in black ink that reads "Gregory Simon".



The forum is supported by dedicated members and staff who have contributed to the immense body of work that the forum has produced over the past 10 years.



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YEARS



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Forum Themes and Priorities

The forum seeks to address the key challenges and opportunities in the discovery, development, and translation of new therapeutics for patients.

The forum seeks to address the key challenges and opportunities in the discovery, development, and translation of new therapeutics for patients, covering the full 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 identified four core components across the R&D lifecycle, which serve as thematic pillars to frame the forum's focus areas and activities: (1) Innovation and the Drug Research and Development Enterprise; (2) Science Across the Biomedical Research Lifecycle (Basic, Translational, and Regulatory Sciences); (3) Clinical Trials and Medical Product Development; and (4) Infrastructure and Workforce for Advancing Drug Discovery, Development, and Translation.

Innovation and the Drug Research and Development Enterprise

Innovative paradigms and new methodologies are needed to bridge the ever-widening gap between scientific discoveries and the translation of those discoveries into life-changing medications. The forum offers a neutral space for stakeholders to advance critical policy discussions on biopharmaceutical research and development nationally and globally.

Science Across the Biomedical Research Lifecycle

Revolutionary advances in biomedical research and technology applications present new and exciting opportunities for the discovery and development of new therapies for patients. The forum highlights critical issues across the biomedical research lifecycle and facilitates the exploration of more person-centric approaches to spur innovation.

Clinical Trials and Medical Product Development

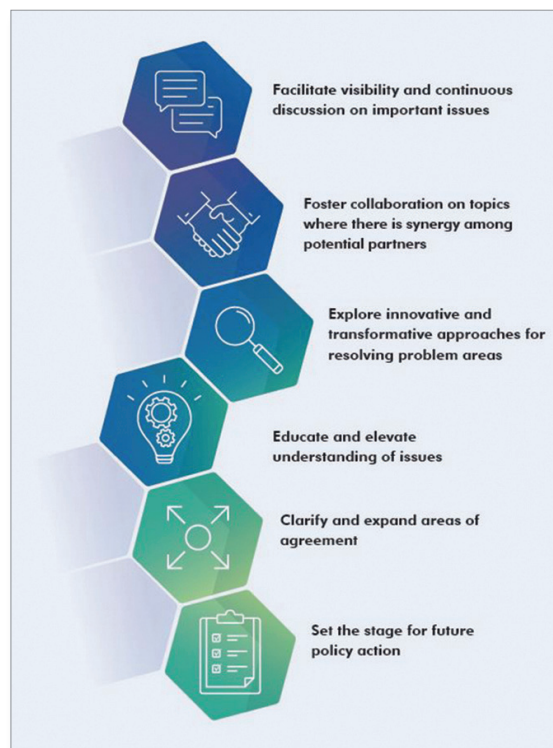
The clinical trials enterprise faces challenges on all sides—rising costs, an evolving regulatory and economic landscape, increasing clinical trial complexity, difficulties in engaging research participants, and a strained workforce. The forum brings together patients, providers, academia, industry, federal agencies, payers, nonprofit organizations, and funders to help overcome these challenges.

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

Considerable opportunities remain to improve and enhance the infrastructure supporting the clinical trials enterprise. The forum has fostered the development of strategies to improve the discipline of innovative regulatory science and continues to focus attention on critical infrastructure and workforce issues.

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 strategies outlined in the image to the right.



Reflecting Back: Forum Activities in 2011–2020

Over the past decade, tremendous advances have been made to improve public participation throughout the drug R&D lifecycle.

Envisioning a Transformed Clinical Trials Enterprise in the United States: Establishing an Agenda for 2020 (2012)¹

In November 2011, amid growing concerns about the future of the U.S. clinical trials enterprise and its competitiveness on the global stage, the forum convened a public workshop for stakeholders to lay out a vision for a transformed clinical trials enterprise in the United States by 2020. At the time, there was growing recognition that the clinical trials enterprise in the United States faced substantial challenges impeding the efficient and effective conduct of clinical research to support the development of new medicines and evaluate existing therapies.

Reflecting back over the past decade, there have been advances in the field as well as setbacks:

Integrating Community Practice and Clinical Research

Despite efforts on the part of key stakeholders, including researchers, sponsors, government agencies, clinicians, and patients, the divide between community practice and clinical research remains. At the time of the workshop, health care was moving toward more integrated delivery systems and the widespread use of EHRs. Workshop participants called for disruptive innovation and a push for health delivery systems—along with other research, clinical, and educational entities—to develop and implement business plans that integrate research into routine clinical practice. Yet today, progress toward this goal has been limited.

¹The full text of *Envisioning a Transformed Clinical Trials Enterprise in the United States: Establishing an Agenda for 2020: Workshop Summary* (IOM, 2012) is available at <https://www.nap.edu/catalog/13345>.



Improving Public Participation in Clinical Trials

Over the past decade, tremendous advances have been made to improve public participation throughout the drug R&D lifecycle, including several laws that incorporate the voice and perspective of patients. In 2010, the Patient-Centered Outcomes Research Institute (PCORI) was established by an act of Congress to improve the quality and relevance of patient-centered evidence available to patients, caregivers, clinicians, policy makers, and other stakeholders for making better informed health decisions. The 2012 FDA Safety and Innovation Act² and corresponding fifth authorization of the Prescription Drug User Fee Act (PDUFA V) included provisions to enhance the role of the patient voice in the drug development process. Within the past few years, Congress passed the 21st Century Cures Act,³ and the FDA Reauthorization Act of 2017,⁴ which included patient engagement provisions. The push for a more people-centered approach to drug R&D has led to a culture shift among public and private stakeholders. “Patient-centeredness” and “patient-focused” drug development are now part of the everyday lexicon.

Forum members at the 42nd forum meeting held in March 2019.

²Food and Drug Administration Safety and Innovation Act of 2012, Public Law 112-144, 112th Cong. (July 9, 2012)

³21st Century Cures Act of 2016, Public Law 114-255, 114th Cong. (Dec. 13, 2016)

⁴FDA Reauthorization Act of 2017, Public Law 115-52, 115th Cong. (Aug. 18, 2017)



Participants discuss actions stakeholders could take to improve transparent reporting of biomedical research during a 2019 workshop on Enhancing Scientific Reproducibility Through Transparent Reporting.

Aligning Cultural and Financial Incentives

There has been significant change in the regulatory environment over the past 10 years. A modernized way of thinking in this space has improved the field of clinical trials. There has been progress when it comes to developing and implementing incentives for drug development to treat rare diseases and cancer. These improved business models have addressed unmet needs for these conditions/diseases, but now the trials enterprise is seeing comparatively low investment in drug innovation for treating prevalent chronic conditions. With many suboptimal drugs for these conditions on the market, the current fee-for-service reimbursement system provides little incentive for the biopharmaceutical industry to invest in developing new treatments. Without sufficient investment, much of the basic biology of these diseases remains unknown, and fewer exciting breakthroughs and opportunities for publishing are furthering the waning interest in researching these conditions.

Building the Infrastructure and Workforce

The clinical trials infrastructure requires substantial resources to recruit investigators, coordinate across research sites, implement quality control systems, enroll and retain trial participants, and seek regulatory approval. Responsible sharing of clinical trial data is in the public interest and helps maximize the benefits of research for future patients and society as a whole. Collaborative approaches are needed to support the collection, management, analysis, and sharing of clinical trial data. The development and application of regulatory science calls for a well-trained, scientifically engaged, and motivated workforce. The forum has fostered the development of strategies to improve the discipline of innovative regulatory science and continues to focus attention on critical infrastructure and workforce issues. Considerable opportunities remain to improve and enhance the infrastructure supporting the clinical trials enterprise.

Clinical Trials and Medical Product Development

Sharing Clinical Research Data—A Workshop (2012)⁵

On **October 4–5, 2012**, four convening bodies—the Forum on Drug Discovery, Development, and Translation; the Forum on Neuroscience and Nervous System Disorders; the National Cancer Policy Forum; and the Roundtable on Translating Genomic-Based Research for Health (now called the Roundtable on Genomics

and Precision Health)—collectively hosted a workshop to examine and advance the sharing of clinical data. The workshop explored the benefits of sharing clinical research data, the barriers to such sharing, and strategies to address these barriers to facilitate the development of safe, effective therapeutics and diagnostics. Participants in the workshop discussed the importance of developing mutually beneficial partnerships in promoting data sharing, the need for data standardization to improve reliability, and how incentives are fundamental to a culture shift that recognizes the value of data sharing.

Sharing Clinical Trial Data—An Action Collaborative⁶

A related action collaborative jointly convened by the Forum on Drug Discovery, Development, and Translation; the Forum on Neuroscience and Nervous System Disorders; the National Cancer Policy Forum; and the Roundtable on Genomics and Precision Health was launched in 2016. Through meetings with interested parties, including nonprofit funders of research, patient advocacy organizations, and data-sharing stakeholders, the action collaborative developed and refined a statement of data-sharing goals that could be incorporated into participating organizations' funding policies. In March 2021, participants published a Viewpoint in the *Journal of Participatory Medicine*, "Data Sharing Goals for Nonprofit Funders of Clinical Trials."⁷

Real-World Evidence Generation and Evaluation of Therapeutics— A Workshop (2016)⁸

On **October 19, 2016**, the forum convened a workshop to examine opportunities and challenges for incorporating real-world evidence into the evaluation of medical products. Workshop participants discussed how various sources of real-world data (e.g., claims data, patient-generated data) could be used to address unmet stakeholder needs; priorities for improving evidence generation to support decision making; barriers to incorporating real-world evidence into product development and process evaluation; and next steps to expand the role of real-world evidence in the development and evaluation of therapeutics.

Examining the Impact of Real-World Evidence on Medical Product Development—A Workshop Series (2017–2018)⁹

Over the course of several months in 2017 and 2018, the forum hosted a series of three workshops on the generation and utilization of real-world evidence, sponsored by FDA. Each workshop in the series focused on a different topic, with workshop 1 exploring how to align incentives to support the collection of real-world data and the use of real-world evidence in health product review, payment, and delivery. Workshop 2 focused on illuminating what types of data are appropriate for what specific purposes and suggesting approaches for data collection and evidence use by developing and working through example use cases. Workshop 3 examined and suggested approaches for operationalizing real-world data collection and the use of real-world evidence.

In June 2021, some individual workshop speakers and attendees published a set of four articles in *Clinical Pharmacology & Therapeutics*, which were inspired by what they learned at this workshop series and took into account lessons learned from the COVID-19 pandemic.

- “When Can We Rely on Real-World Evidence to Evaluate New Medical Treatments?” outlines the paper series’ scope, context, and the dimensions considered where real-world evidence studies may differ from traditional trials.¹⁰
- “When Can We Trust Real-World Data to Evaluate New Medical Treatments?” lays out questions to help stakeholders assess potential error or bias at each step of data generation from clinical presentation (including with a device) to inclusion in a database.¹¹

⁵The full text of *Sharing Clinical Research Data: Workshop Summary* (IOM, 2013) is available at <https://www.nap.edu/catalog/18267>.

⁶For more information on this action collaborative, see <https://www.nationalacademies.org/our-work/sharing-clinical-trial-data-an-action-collaborative>.

⁷The article “Data Sharing Goals for Nonprofit Funders of Clinical Trials” (2021) published in the *Journal of Participatory Medicine* is available at <https://jopm.jmir.org/2021/1/e23011>.

⁸The full text of *Real-World Evidence Generation and Evaluation of Therapeutics: Proceedings of a Workshop* (NASEM, 2017) is available at <https://www.nap.edu/catalog/24685>.

⁹The full text of *Examining the Impact of Real-World Evidence on Medical Product Development: Proceedings of a Workshop* (NASEM, 2019) is available at <https://www.nap.edu/catalog/25352>.

¹⁰The article “When Can We Rely on Real-World Evidence to Evaluate New Medical Treatments?” (2021) published in *Clinical Pharmacology & Therapeutics* is available at <https://ascpt.onlinelibrary.wiley.com/doi/10.1002/cpt.2253>.

¹¹The article “When Can We Trust Real-World Data to Evaluate New Medical Treatments?” (2021) published in *Clinical Pharmacology & Therapeutics* is available at <https://ascpt.onlinelibrary.wiley.com/doi/10.1002/cpt.2252>.



Forum member, Ellen Sigal, shares her thoughts at the March 2018 forum meeting.

- “When Are Treatment Blinding and Treatment Standardization Necessary in Real-World Clinical Trials?” lays out questions to help stakeholders determine whether blinding and treatment standardization may be necessary for the internal validity of a study or may distort meaningful differences between treatments in specific cases.¹²
- “When Can Nonrandomized Studies Support Valid Inference Regarding Effectiveness or Safety of New Medical Treatments?” lays out questions to help stakeholders evaluate the potential impact of confounding or other biases on their research findings.¹³

Improving the Drug Development Process Through Examining Late-Stage Failures—An Action Collaborative¹⁴

This action collaborative examined the contributing factors in the failure of late-stage clinical product development and developed key considerations for stakeholders to improve the probability of success. Action collaborative participants conducted a literature survey as well as a series of structured informational interviews with stakeholders directly involved in late-stage therapeutic development. Results were analyzed, categorized, and presented at a meeting of action collaborative participants at which additional insights were discussed. The efforts of action collaborative participants culminated in the publication of an article in *Nature Reviews Drug Discovery*, “The Failure to Fail Smartly.”¹⁵

Innovation and the Drug Research and Development Enterprise Virtual Clinical Trials: Challenges and Opportunities— A Workshop (2018)¹⁶

On **November 28–29, 2018**, the forum hosted a workshop, Virtual Clinical Trials: Challenges and Opportunities. This workshop examined the clinical trials infrastructure and potential opportunities for supporting the practical implementation of virtual clinical trials. Workshop participants discussed the inefficiencies of the existing clinical trial enterprise; the boundaries of what might be considered a virtual clinical trial; opportunities to expand access for patients; perspectives of using digital health technologies in clinical care and observational and interventional studies; impact of using digital health technologies on access and equity to clinical trials; policy landscape governing clinical trials; and possible opportunities for future action.

¹²The article “When Are Treatment Blinding and Treatment Standardization Necessary in Real-World Clinical Trials?” (2021) published in *Clinical Pharmacology & Therapeutics* is available at <https://ascpt.onlinelibrary.wiley.com/doi/10.1002/cpt.2256>.

¹³The article “When Can Nonrandomized Studies Support Valid Inference Regarding Effectiveness or Safety of New Medical Treatments?” (2021) published in *Clinical Pharmacology & Therapeutics* is available at <https://ascpt.onlinelibrary.wiley.com/doi/10.1002/cpt.2255>.

¹⁴For more information on this action collaborative, see <https://www.nationalacademies.org/our-work/improving-the-drug-development-process-through-examining-late-stage-failures-an-action-collaborative>.

¹⁵The article “The Failure to Fail Smartly” (2020) published in *Nature Reviews Drug Discovery* is available at <https://www.nature.com/articles/d41573-020-00167-0>.

¹⁶The full text of *Virtual Clinical Trials: Challenges and Opportunities: Proceedings of a Workshop* (NASEM, 2019) is available at <https://www.nap.edu/catalog/25502>.

There is growing momentum to incorporate patient input into medical product R&D and regulatory decision-making processes.

Mapping and Connecting the Biomedical Innovation Ecosystem—An Action Collaborative¹⁷

The Mapping and Connecting the Biomedical Innovation Ecosystem action collaborative was established under the auspices of the forum to help frame, map, and synergize activities and communication across the biomedical innovation ecosystem. Action collaborative participants developed two process maps—coined the Drug Discovery, Development, and Deployment Maps (4DM)—that diagram the development of small molecules and biologics, which are digitally hosted by the National Center for Advancing Translational Sciences at NIH. The process maps served as the foundation for two complementary peer-reviewed articles released in December 2017 and co-authored by action collaborative participants to guide future dialogue and progress on this topic. “A Dynamic Map for Learning, Communicating, Navigating, and Improving Therapeutic Development” was published in *Nature Reviews Drug Discovery*¹⁸ and “Application of a Dynamic Map for Learning, Communicating, Navigating, and Improving Therapeutic Development” was simultaneously published in *Clinical and Translational Science*.¹⁹

The Role of Digital Health Technologies in Drug Development—A Workshop (2020)²⁰

The forum and the Roundtable on Genomics and Precision Health hosted a public workshop on **March 24, 2020**, which provided a venue for stakeholders to discuss challenges and opportunities in using digital health technologies to improve the probability of success in drug development. The COVID-19 pandemic, which was only beginning to disrupt daily life at the time of the workshop, added a sense of urgency to the workshop discussions on practical and ethical considerations for the development and implementation of digital health technologies. Workshop participants considered key components for an evidence-based framework for applying digital health technologies toward drug R&D. Workshop participants discussed how digital health technologies could be uniquely suited to address persistent problems in drug R&D, considered barriers to implementing digital health technologies, and discussed opportunities to move the field forward.



Forum member, Freda Lewis-Hall, presents her thoughts during a discussion at the March 2018 forum meeting.

¹⁷For more information on this action collaborative, see <https://www.nationalacademies.org/our-work/mapping-and-connecting-the-biomedical-innovation-ecosystem-an-action-collaborative>.

¹⁸The article "A Dynamic Map for Learning, Communicating, Navigating, and Improving Therapeutic Development" (2017) published in *Nature Reviews Drug Discovery* is available at <https://www.nature.com/articles/nrd.2017.217>.

¹⁹The article "Application of a Dynamic Map for Learning, Communicating, Navigating, and Improving Therapeutic Development" (2017) published in *Clinical and Translational Science* is available at <https://ascpt.onlinelibrary.wiley.com/doi/full/10.1111/cts.12531>.

²⁰The full text of *The Role of Digital Health Technologies in Drug Development: Proceedings of a Workshop* (NASEM, 2020) is available at <https://www.nap.edu/catalog/25850>.

Science Across the Biomedical Research Lifecycle

Deriving Drug Discovery Value from Large-Scale Genetic Bioresources—A Workshop (2016)²¹

On **March 22, 2016**, the forum and the Roundtable on Genomics and Precision Health convened a workshop to examine and discuss how large-scale genetic data could be used to improve the likelihood of bringing effective and targeted therapies to patients. Workshop participants assessed the current landscape of genomic-enabled drug discovery and development activities; examined enabling partnerships and better business models; and considered gaps and best practices in how data from populations could be collected with the goal of improving the drug discovery process.

Enabling Precision Medicine: The Role of Genetics in Clinical Drug Development—A Workshop (2018)²²

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 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 a workshop that 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 took 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 considered how these experiences might inform the advancement of precision medicine.



The forum hosts speakers at the workshop on Drug Research and Development for Adults Across the Older Age Span, held virtually during the COVID-19 pandemic in 2020.

Enhancing Scientific Reproducibility in Biomedical Research Through Transparent Reporting—A Workshop (2019)²³

In collaboration with the Forum on Neuroscience and Nervous System Disorders, the National Cancer Policy Forum, and the Roundtable on Genomics and Precision Health, the forum convened a workshop on **September 25–26, 2019**, to examine the state of transparency in reporting biomedical research (e.g., disclosure of the availability and location of data, materials, analyses, and methodology) and to explore the possibility of improving the harmonization of guidelines across journals and funding agencies so that biomedical researchers propose and report data in a consistent manner. Workshop participants discussed the challenges and opportunities for the harmonization of guidelines for transparent reporting throughout the biomedical research lifecycle. The agenda included a panel discussion on facilitating the development of consistent guidelines (e.g., a common set of minimal reporting standards) that could be applied across journals and funders to increase transparency in proposing and reporting biomedical research. This workshop was sponsored by Cell Press, *The Lancet*, Nature Research, and NIH.

²¹The full text of *Deriving Drug Discovery Value from Large-Scale Genetic Bioresources: Proceedings of a Workshop* (NASEM, 2016) is available at <https://www.nap.edu/catalog/23601>.

²²The full text of *Enabling Precision Medicine: The Role of Genetics in Clinical Drug Development: Proceedings of a Workshop* (NASEM, 2017) is available at <https://www.nap.edu/catalog/24829>.

²³The full text of *Enhancing Scientific Reproducibility in Biomedical Research Through Transparent Reporting: Proceedings of a Workshop* (NASEM, 2020) is available at <https://www.nap.edu/catalog/25627>.



Forum members (from left to right) Ross McKinney, Jr., Anantha Shekhar, Bernard Munos, and Ann E. Taylor sharing their thoughts at the July 2019 forum meeting.

Person-centeredness and Inclusivity

Advancing the Science of Patient Input on Medical Product R&D: Towards a Research Agenda—A Workshop (2018)²⁴

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 could better align medical product development and regulation with patient perspectives on disease experience, burden, management, and treatment. The forum convened a 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 examined the current state of the science, including successes and limitations of existing efforts, for soliciting and incorporating patient input in pre-market R&D; explored gaps in the knowledge base and other barriers that impede progress; and reviewed a potential framework for and components of a research agenda for addressing the gaps or barriers to realizing a science of patient input.

Drug Research and Development for Adults Across the Older Age Span—A Workshop (2020)²⁵

Despite the widespread recognition of the “graying of America,” and the need for health care among older adults, there is a dearth of information about the appropriate use of drugs in this population. Older adults are vastly underrepresented in clinical trials. Yet, older adults have higher rates of comorbidities and polypharmacy than the general population and are the majority users of many medications. Additionally, age-related physiological and pathological changes, particularly for adults over age 80, can lead to significant differences in the pharmacokinetic and pharmacodynamics of a given drug compared to the general population. There is a void in evidence-based information for making informed decisions on how to best optimize care for older adults. In collaboration with the Forum on Aging, Disability, and Independence and the National Cancer Policy Forum, the forum hosted a workshop on **August 5 and 6, 2020**,

²⁴The full text of *Advancing the Science of Patient Input in Medical Product R&D: Towards a Research Agenda: Proceedings of a Workshop—in Brief* (NASEM, 2018) is available at <https://www.nap.edu/catalog/25325>.

²⁵The full text of *Drug Research and Development for Adults Across the Older Age Span: Proceedings of a Workshop* (NASEM, 2021) is available at <https://www.nap.edu/catalog/25998>.

The development and application of regulatory science . . . calls for a well-trained, scientifically engaged, and motivated workforce.

that discussed the challenges and opportunities in drug R&D for the over age 65 and over age 80 populations, explored barriers that impede safety and efficacy studies in these populations, and shared lessons learned and best practices for better understanding the clinical pharmacology for older adults.

Infrastructure and Workforce

Building a National Framework for the Establishment of Regulatory Science for Drug Development—A Workshop (2010)²⁶

The 2007 Institute of Medicine (IOM) report *The Future of Drug Safety: Promoting and Protecting the Health of the Public*²⁷ identified the need for an improved science base for drug evaluation within FDA, including internal resources and extramural funding for collaboration with academia. In that same year, the FDA Science Board,²⁸ at the request of Congress, reported on the agency's need for an enhanced science base, including infrastructure development, multisector collaboration, and an expanded workforce capable of addressing the rapidly evolving science of drug discovery and development. In 2008, the forum held a public workshop to explore the science of drug regulation, focusing on the gap between leading-edge technologies of drug development and FDA's capacity to adapt its process of regulatory evaluation to these technologies. Together, the results of these efforts suggested a widening gap between scientific developments in areas relevant to FDA's mission and its ability to address these innovations, as well as a lack of understanding among the public, policy makers, and the agency of what is required to fill this gap. To address these concerns, the forum convened a public workshop on **February 26, 2010**, to examine the state of the science of drug regulation and consider approaches for enhancing the scientific basis of regulatory decision making. Workshop participants explored the concept of regulatory science, examined how it could be used to improve regulatory decision making, and considered alternative mechanisms and institutional frameworks for its development and application.

Strengthening a Workforce for Innovative Regulatory Science in Therapeutics Development—A Workshop (2011)²⁹

The development and application of regulatory science—which FDA defined as the science of developing new tools, standards, and approaches to assess the safety, efficacy, quality, and performance of FDA-regulated products—calls for a well-trained, scientifically engaged, and motivated workforce. FDA faces challenges in retaining regulatory scientists and providing them with opportunities for professional development. In the private sector, advancement of innovative regulatory science in drug development has not always been clearly defined, well-coordinated, or connected to the needs of the agency. On **September 20–21, 2011**, the forum held a public workshop that considered the opportunities and needs for advancing innovation in the discipline of regulatory science for therapeutics development through an interdisciplinary regulatory science workforce. Workshop participants discussed the core components of a robust discipline of regulatory science; deliberated on the key competencies for a regulatory science workforce; and examined the needs and opportunities to promote training, career development, and collaborative approaches to sustain and nurture a workforce in innovative regulatory science.

²⁹The full text of *Building a National Framework for the Establishment of Regulatory Science for Drug Development: Workshop Summary* (IOM, 2011) is available at <https://www.nap.edu/catalog/12968>.

²⁷The full text of *The Future of Drug Safety: Promoting and Protecting the Health of the Public* (IOM, 2007) is available at <https://www.nap.edu/catalog/11750>.

²⁸The FDA Science Board is an advisory committee with the following mission: "The Science Board shall provide advice to the Commissioner and other appropriate officials on specific complex scientific and technical issues important to FDA and its mission, including emerging issues within the scientific community. Additionally, the Science Board will provide advice that supports the Agency in keeping pace with technical and scientific developments, including in regulatory science; and input into the Agency's research agenda; and on upgrading its scientific and research facilities and training opportunities. It will also provide, where requested, expert review of Agency sponsored intramural and extramural scientific research programs."

²⁸The full text of *Strengthening a Workforce for Innovative Regulatory Science in Therapeutics Development: Workshop Summary* (IOM, 2012) is available at <https://www.nap.edu/catalog/13283>.

Participants of The Global Crisis of Drug-Resistant Tuberculosis in BRICS Countries workshop hosted in New Delhi, India, in 2011.



Special Topics

The Global Crisis of Drug-Resistant Tuberculosis in BRICS Countries— A Workshop Series (2008–2013)³⁰

The forum convened a multiyear, international initiative on multidrug-resistant tuberculosis (MDR TB), which began with a foundational workshop in Washington, DC, and included international workshops in the high-burden countries of South Africa, Russia, India, and China. To effectively treat patients diagnosed with MDR TB and protect populations from further transmission of the disease, an uninterrupted supply of quality-assured, second-line, anti-TB drugs (SLDs) is necessary.

Workshop participants discussed issues related to the global drug supply chain for quality-assured SLDs for MDR TB; explored innovative solutions to delivering the right treatments to people who critically need them (e.g., strategies to improve logistics, supply and demand, quality of SLDs, and financing arrangements for the global supply chain); highlighted key challenges to controlling disease spread; and discussed innovative strategies to advance and harmonize local and international efforts to prevent and treat MDR TB.

Enabling Rapid Response and Sustained Capability with Medical Countermeasures to Mitigate the Risk of Emerging Infectious Diseases (2015)³¹

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 the production of vaccines, therapeutics, diagnostic tools, and other non-pharmaceutical interventions. On **March 26–27, 2015**, the forum, in collaboration with the Forum on Medical and Public Health Preparedness for Catastrophic Events (now called the Forum on Medical and Public Health Preparedness for Disasters and Emergencies) and the Forum on Microbial Threats, 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 the policies, guidance, and available resources set forth by the U.S. government. The workshop also explored advances made by the Public Health Emergency Medical Countermeasures Enterprise to improve MCM development and translation.

³⁰The summaries of these workshops are available at:

- *Addressing the Threat of Drug Resistant Tuberculosis: Workshop Summary* (IOM, 2009): <https://www.nap.edu/catalog/12570>.
- *The Emerging Threat of Drug-Resistant Tuberculosis in Southern Africa—Workshop Summary* (2011): <https://www.nap.edu/catalog/12993/the-emerging-threat-of-drug-resistant-tuberculosis-in-southern-africa>.
- *The New Profile of Drug-Resistant Tuberculosis in Russia: Workshop Summary* (IOM, 2011): <https://www.nap.edu/catalog/13033>.
- *Facing the Reality of Drug-Resistant Tuberculosis in India: Workshop Summary* (IOM, 2012): <https://www.nap.edu/catalog/13243>.
- *Developing and Strengthening the Global Supply Chain for Second-Line Drugs for Multidrug-Resistant Tuberculosis: Workshop Summary* (IOM, 2013): <https://www.nap.edu/catalog/13524>.
- *The Global Crisis of Drug-Resistant Tuberculosis and Leadership of China and the BRICS: Workshop Summary* (IOM, 2014): <https://www.nap.edu/catalog/18346>.

³¹The full text of *Rapid Medical Countermeasure Response to Infectious Diseases: Enabling Sustainable Capabilities Through Ongoing Public- and Private-Sector Partnerships: Workshop Summary* (NASEM, 2016) is available at <https://www.nap.edu/catalog/21809>.

Looking Forward:

Forum Activities in 2021 and Beyond

Forum Membership Meetings

In 2021, the forum membership will 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 identified priority areas, including the activities outlined in the following sections.

The biological, physical, and digital spheres are merging; clinical research and health care are at a critical juncture; and new technologies enable the collection of data in real-world settings.

Envisioning a Transformed Clinical Trials Enterprise: Establishing an Agenda for 2030³²

Clinical trials research has changed dramatically over the past decade. The biological, physical, and digital spheres are merging; clinical research and health care are at a critical juncture; and new technologies enable the collection of data in real-world settings. These opportunities hold great promise for advancing our understanding of health maintenance and prevention, disease progression, and developing new therapies for patients. At the same time, the clinical research enterprise faces continued and mounting pressures, strained from all sides by rising costs, an evolving regulatory and economic landscape, increasing clinical trial complexity, difficulties in the recruitment and retention of research participants, and a clinical research workforce that is under tremendous stress. Some, but not all, of these challenges and opportunities were predicted in the 2011 forum-hosted workshop *Envisioning a Transformed Clinical Trials Enterprise in the United States: Establishing an Agenda for 2020*. There is now a need for stakeholders from across the clinical research lifecycle, including patients, caregivers, sponsors, technologists, regulators, practitioners, and researchers, to examine the challenges and setbacks, lessons learned, and advances from the past decade and consider the challenges



Forum members Tanisha Carino and Luther Clark chat after the March 2019 forum meeting.

and opportunities that may be coming over the next 10 years. On **January 26, February 9, March 24, and May 11, 2021**, the forum will convene a public workshop to discuss how to better integrate community practice and clinical trials, improve patient engagement in clinical trials, align cultural and financial incentives, and better support the clinical trials infrastructure. The workshop will provide a venue to consider a transformed clinical trial enterprise for 2030 and discuss the goals and key priorities for advancing a clinical trials enterprise that is more efficient, effective, person-centered, inclusive, and integrated into the health care delivery system of 2030

Improving the Evidence Base for Treating Older Adults with Cancer³³

As the U.S. population ages, the overall number of individuals diagnosed with cancer is increasing and older adults also account for a growing proportion of both

³²For more information and updates on this project, see <https://www.nationalacademies.org/our-work/envisioning-a-transformed-clinical-trials-enterprise-for-2030-a-workshop>.

³³For more information and updates on this project, see <https://www.nationalacademies.org/event/01-22-2021/improving-the-evidence-base-for-treatment-decision-making-for-older-adults-with-cancer-a-workshop>.

cancer diagnoses and cancer deaths. Clinicians, along with patients with cancer, and their families rely on clinical research for information on the potential benefits and harms of different treatment options so that they can make informed treatment decisions that are consistent with the patients' needs, values, and preferences. However, the evidence base for older adults with cancer is limited by a pervasive underrepresentation of this population in cancer clinical trials. To begin to address these issues, the forum, along with the National Cancer Policy Forum and the Forum on Aging, Disability, and Independence, will host an FDA-sponsored, public workshop on **January 22, 25, and 27, 2021**. This workshop will aim to illuminate a path forward in generating meaningful evidence for the treatment of older adults with cancer.

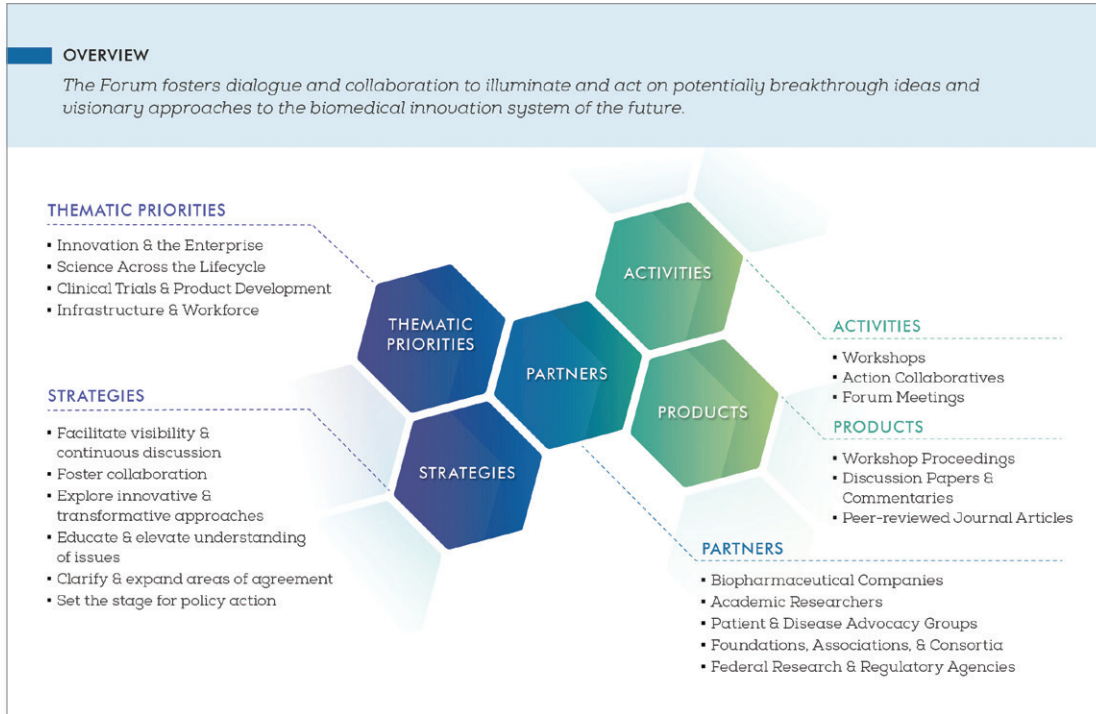
Innovation in Drug Research and Development for Prevalent Chronic Diseases³⁴

Chronic diseases such as heart disease, stroke, cancer, and diabetes are the leading cause of death and disability among Americans and a major factor in the nation's rapidly increasing health care costs. At present, 6 in 10 U.S. adults live with at least one chronic disease, and 4 in 10 have two or more. However, despite the human and financial costs of chronic diseases, investment in the disorders has not kept pace with the need. On **February 22 and March 1 and 8, 2021**, the forum will host a public workshop to examine and highlight the bottlenecks to innovation in drug R&D for prevalent chronic diseases and consider the challenges and opportunities for spurring drug R&D in this space.

Accelerating the Development of Rapid Diagnostics to Address Antibiotic Resistance

The use and misuse of antibiotics contributes to the rise in drug-resistant bacteria—a serious and worsening threat to human health. Addressing the problem of antibiotic resistance requires measures to spur innovation and ensure the prudent use of existing drugs. Rapid diagnostics can play an important role in avoiding the unnecessary use of antimicrobials by providing clinicians with the right information at the right time to help them make decisions about appropriate drug treatment

³⁴For more information and updates on this project, see <https://www.nationalacademies.org/event/02-22-2021/innovation-in-drug-research-and-development-for-prevalent-chronic-diseases-a-virtual-workshop>.



for patients, have the capacity to support early detection and diagnosis of drug-resistant bacterial infections, enable disease surveillance, and help prevent disease spread. Participants in this public workshop will discuss the current landscape of rapid diagnostics to address antibiotic resistance, consider the challenges and opportunities for spurring innovation, and discuss practical next steps for accelerating the development of new diagnostic tools.

Action collaboratives are ad hoc, participant-driven activities associated with the forum that foster collaboration and information sharing among forum members and external thought leaders and stakeholders.

Action Collaboratives

Action collaboratives are ad hoc, participant-driven activities associated with the forum that foster collaboration and information sharing among forum members and external thought leaders and stakeholders. These activities engage experts with similar interests and responsibilities to analyze in-depth, high-priority issues and to advance the identified goals of the forum and recommendations highlighted in National Academies' Consensus Study Reports.

Clinical Trial Site Standards Harmonization³⁵

The cost and duration of clinical trials have escalated dramatically in the past 20 years, hampering the introduction of new therapies; driving recruitment to developing countries; and raising questions about generalizability, data quality, and participant protections. While some sites in the United States are extremely efficient in launching new trials and in identifying participants, many still suffer from long delays in contracting and Institutional Review Board approvals, and enrollment targets are frequently missed. The protections of research participants are variably applied, with the processes for consent and adherence to Good Clinical Practice suboptimal at some sites, and standards for training and site-level preparedness are variable. The consequences are borne by NIH, foundations, and industry alike, with the ultimate impact on the public.

This action collaborative seeks to accelerate progress in the improvement of the national clinical trials infrastructure. To date, the action collaborative has drawn together a group of diverse stakeholders for four in-person meetings (December 2012, August 2013, March 2014, and September 2018). Action collaborative participants published a discussion paper,³⁶ which summarized their perspectives on the concept of clinical trial site accreditation and laid out a framework and principles for developing clinical trial site standards. In addition, the action collaborative has undertaken an activity to collect, analyze, and prioritize a set of core site standards that could be considered for broad application by research sponsors.



Left to right: Current Forum Co-Chair, Robert Califf, former Forum Director Anne Claiborne, and former Forum Co-Chairs Russ Altman and Steven Galson during the June 2015 forum meeting.

Science of Patient Input³⁷

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/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. Many efforts have been launched to advance a science of patient input. However, there is a critical need to identify gaps in the knowledge base and other barriers that impede progress and develop a research agenda for addressing them.

This action collaborative was established to identify gaps in the knowledge base and other barriers that impede progress in advancing the science of patient input. Stakeholders came together for a meeting of the action collaborative on July 30, 2019, to discuss and prioritize gaps and barriers that should be included in a research agenda to advance the science of patient input. In 2021, action collaborative participants will work toward the publication of this activity.

³⁶For more information and updates on this project, see <https://www.nationalacademies.org/our-work/clinical-trial-site-standards-harmonization-an-action-collaborative>.

³⁶The discussion paper “It’s Time to Harmonize Clinical Trial Site Standards” (2017) published in *NAM Perspectives* is available at <https://nam.edu/its-time-to-harmonize-clinical-trial-site-standards>.

³⁷For more information and updates on this project, see <https://www.nationalacademies.org/our-work/advancing-the-science-of-patient-input-in-medical-product-rd-an-action-collaborative>.

Innovation in Drug Research and Development for Highly Prevalent Chronic Diseases³⁸

Half of all Americans live with at least one chronic disease,³⁹ such as heart disease, cancer, stroke, or diabetes. These and other chronic diseases are the leading cause of death and disability in the United States and are a leading driver of health care costs, with approximately 90 percent of health care expenditures related to chronic conditions.^{40,41} Yet, investment in these leading causes of death and disability, other than cancer, has not reflected this burden. The 2020 outbreak of coronavirus disease 2019 (COVID-19) may further exacerbate the health disparities associated with high-impact prevalent chronic diseases.

The goal of this action collaborative is to examine and highlight the bottlenecks to drug R&D for highly prevalent chronic diseases and develop a set of key considerations and potential strategies for spurring innovation. In 2021, action collaborative participants will develop and distribute a structured, qualitative questionnaire to key stakeholders. Results will be synthesized and used to inform next steps for the action collaborative.

³⁸For more information and updates on this project, see <https://www.nationalacademies.org/our-work/innovation-in-drug-research-and-development-for-highly-prevalent-chronic-diseases-an-action-collaborative>.
³⁹Broadly defined by the National Center for Chronic Disease Prevention and Health Promotion as "conditions that last 1 year or more and require ongoing medical attention or limit activities of daily living or both."

See <https://www.cdc.gov/chronicdisease/about/index.htm>.

⁴⁰See <https://www.cdc.gov/chronicdisease/index.htm>.

⁴¹Buttorff, C., T. Ruder, and M. Bauman. 2017. *Multiple Chronic Conditions in the United States*. Santa Monica, CA: RAND Corporation. See https://www.rand.org/content/dam/rand/pubs/tools/TL200/TL221/RAND_TL221.pdf.



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National Institute of Mental Health, NIH*
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Foundation for the National Institutes of Health*
Friends of Cancer Research*
Howard Hughes Medical Institute
March of Dimes
*New England Journal of Medicine**

MEMBERS

(as of December 2020)

Forum membership 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.

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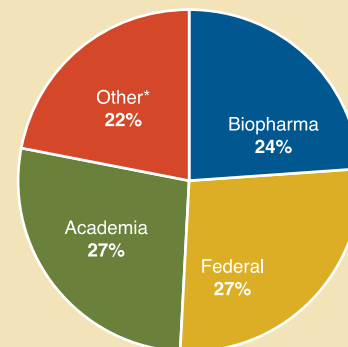
Alastair Wood

Vanderbilt University

Janet Woodcock

Center for Drug Evaluation and Research, FDA

Membership



*Consortia, foundations, journals, patient-focused/disease advocacy organizations.

ACKNOWLEDGMENTS

Thanks goes to the current and former co-chairs of the forum—Edward W. Holmes (2005–2008), Gail H. Cassell (2005–2011), Jeffrey M. Drazen (2008–2013), Steven K. Galson (2011–2017), Russ B. Altman (2015–2018), Robert M. Califf (2018–present), and Gregory E. Simon (2019–present)—for their contributions and leadership. Additional thanks goes to former forum members and advisers as well as former staff who provided thoughtful input, guidance, and support for forum activities and publications.

T I M E L I N E

2020 | March 24 Workshop: The Role of Digital Health Technologies in Drug Development (in collaboration with the Roundtable on Genomics and Precision Health) | **March 25** Forum Meeting #45 | **July 8–9** Forum Meeting #46 | **August 5–6** Drug R&D for Adults Across the Older Age Span (in collaboration with the Forum on Aging, Disability, and Independence and the National Cancer Policy Forum) | **October 29** Forum Meeting #47

2019 | March 6–7 Forum Meeting #42 | **March 26** Meeting: Updating Labels for Generic Oncology Drugs (in collaboration with the National Cancer Policy Forum) | **July 30–31** Forum Meeting #43 | **September 25–26** Workshop: Enhancing Scientific Reproducibility Through Transparent Reporting (in collaboration with the Forum on Neuroscience and Nervous System Disorders, the National Cancer Policy Forum, and the Roundtable on Genomics and Precision Health) | **October 23–24** Forum Meeting #44 | **November 18–19** Workshop: Sharing Clinical Trial Data: Challenges and a Way Forward (in collaboration with the Forum on Neuroscience and Nervous System Disorders, the National Cancer Policy Forum, and the Roundtable on Genomics and Precision Health)

2018 | March 6–7 Workshop Series: Examining the Impact of Real-World Evidence on Medical Product Development—Workshop 2: Practical Approaches | **March 19–20** Forum Meeting #39 | **May 9** Workshop: Advancing the Science of Patient Input in Medical Product R&D: Towards a Research Agenda | **July 17–18** Workshop Series: Examining the Impact of Real-World Evidence on Medical Product Development—

Workshop 3: Application | **July 18** Forum Meeting #40 | **October 3–4** Forum Meeting #41 | **November 28–29** Workshop: Virtual Clinical Trials: Challenges and Opportunities

2017 | March 8 Workshop: Enabling Precision Medicine: The Role of Genetics in Clinical Drug Development (in collaboration with the Roundtable on Genomics and Precision Health) | **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

2016 | March 12 Workshop: Deriving Drug Discovery Value from Large-Scale Genetic Bioresources (in collaboration with the Roundtable on Genomics and Precision Health) | **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)

2015 | January 20–21 Workshop: Financial Incentives to Support Unmet Medical Needs for Nervous System Disorders (in collaboration with the Forum on Neuroscience and Nervous System Disorders) | **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 Forum on Medical and Public Health Preparedness for

Disasters and Emergencies) | **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

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

2013 | **January 15** Workshop Summary 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

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 Forum on Neuroscience and Nervous System Disorders, the National Cancer Policy Forum, and the Roundtable on Genomics and Precision Health) | **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)

2011 | **March 28** Forum Meeting #18 | **March 29–30** Workshop: Advancing Regulatory Science for Medical Countermeasure Development (in collaboration with the Forum on Medical and Public Health Preparedness for Disasters and Emergencies) | **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 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 Release: *The New Profile of Drug-Resistant Tuberculosis: A Global and Local Perspective* (Moscow, Russia)

T I M E L I N E

2010 | **February 23** Workshop: The Public Health Emergency Medical Countermeasures Enterprise (in collaboration with the Forum on Medical and Public Health Preparedness for Disasters and Emergencies) | **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

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

2008 | **February 20** Discussion Series: Comparative Effectiveness | **February 20–21** 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 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

2007 | **March 12** Symposium: The Future of Drug Safety: Challenges for 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

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

2005 | **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

2002–2004 | Clinical Research Roundtable, predecessor to the forum

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ABOUT THE FORUM

The **Forum on Drug Discovery, Development, and Translation** of the **National Academies of Sciences, Engineering, and Medicine** 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 a few 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>.

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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.

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