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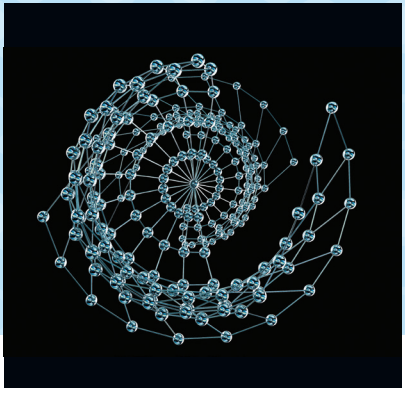


Forum on Drug Discovery, Development, and Translation

2016 Annual Report

Message from the Co-Chairs

Russ Altman and Steven Galson

The Forum on Drug Discovery, Development, and Translation of the National Academies of Sciences, Engineering, and Medicine (the 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 relating to drug discovery, development, and translation. The Forum brings attention and visibility to important issues; explores new approaches for resolving problem areas; helps define the scope of the field and thus sets the stage for future policy action; provides a catalyst for collaboration on topics where there is synergy among potential partners; and elevates the general understanding of drug discovery, development, and translation among the research, public policy, and broader communities. The Forum membership includes leaders from the Food and Drug Administration (FDA) and the National Institutes of Health (NIH), the biopharmaceutical industry, academia, foundations, and patient-focused and disease advocacy organizations. The group is self-governing, with Forum members convening several times each year to identify and prioritize the topics they wish to address.

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

The Forum views challenges as opportunities and has used its convening to address these and a variety of extraordinarily complex issues. In 2016, the Forum provided a focused and neutral venue for stakeholders to identify and characterize the needs and priorities in the drug discovery and development "ecosystem" and to encourage meaningful information sharing and collaboration across sectors. The Forum contributed to broad conversations on drug development research and policy, including convening a workshop to explore a forward-looking agenda for generating and incorporating real-world evidence into the development of therapeutics. The Forum membership also continued its focused effort to address challenges in the drug discovery and development process by facilitating an action-oriented collaborative that identified rate-limiting steps and facilitated dialogue for potential process improvement efforts in the biomedical innovation ecosystem. The Forum also supported and fostered cross-disciplinary dialogue through workshops focusing on genetic bioresources for drug discovery and seamless cancer-focused drug development.

In 2017, a broad and deep policy conversation continues about how we can advance biomedical product innovation nationally and globally. The executive and legislative branches of the federal government have shown initiative in marshalling bipartisan support for biomedical research, including developing and supporting new programs such as the BRAIN Initiative, the Precision Medicine Initiative, the Cancer Moonshot, and the 21st Century Cures Act. As a result, government funders and regulators of biomedical research such as FDA and NIH stand to receive an infusion of funding and support for relevant programmatic priorities.

The Forum is well-positioned to participate in policy discussions to help promote the goals and sustain the momentum spurred by these initiatives as they grow in 2017 and beyond. In addition to continuing action collaborative work on responsible sharing of clinical trial data and harmonization of clinical trial site standards, the Forum will launch new action collaboratives in 2017 focused on addressing the barriers and opportunities to improving the drug development process and on furthering the science of patient input. The Forum will also convene a workshop exploring enabling precision medicine through genetics in clinical drug development. The group will also serve as a center for thought, dialogue, and action to advance the use of real-world evidence in developing and evaluating medical products by convening a workshop and venue for the development of discussion papers on the issue. Through these and other working group discussions and workshops, solicited and original qualitative research and collaborative writing, and broad outreach, the Forum will serve as a hub and catalyst for new ideas and directions.

As a neutral convening venue for stakeholders and collaborators, the Forum provides a unique setting in which complex issues of health science policy can be tackled collegially, and in which partnerships may be formed and nurtured. We look forward to another groundbreaking and productive year for the Forum in 2017.

Russ Altman
Co-Chair

Steven Galson
Co-Chair

Reflecting Back

Forum Activities in 2016

Forum Meetings

The Forum membership met three times in 2016. Discussions at these meetings focused on diverse topics related to the Forum's priorities, including improving drug development through genomics; seamless drug development; mapping and connecting the biomedical innovation ecosystem; overcoming challenges in biomedical innovation; precision medicine; strategies for the responsible sharing of clinical trial data; and policy updates relevant to drug discovery, development, and translation. In addition, the Forum convened public workshops and collaborative activities, described below.

(Left to right) Forum director Anne Claiborne and Forum co-chairs Russ Altman and Steven Galson brainstorm during the October 18 Forum meeting.

FORUM ACTIVITIES TIMELINE

2000–2004

Clinical Research Roundtable, predecessor to the Forum

2005

March 23–24
Forum Meeting #1

June 29–30
Forum Meeting #2

Workshops:

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

Biopharmaceutical companies continually seek innovative strategies to revitalize and create efficiencies in their drug development processes. One approach has involved the adoption of genetically guided strategies to reduce attrition rates and increase the odds of success. Several large cohort studies have incorporated or begun to incorporate genetic data collection as part of the study design. With the large volumes of genetic and phenotypic data that are planned to be collected, these efforts could provide a valuable trove of information for identifying and validating potential targets, elucidating underlying disease and mechanistic biology, and developing biomarker assays and targeted therapies. Questions remain about how large cohort studies could be designed, the types of data that should be collected, and which business models could engage stakeholders most effectively. The Forum, in collaboration with the Roundtable on Translating Genomic-Based Research for Health, convened a workshop that examined and discussed how large-scale genetic data could be used to improve the likelihood of bringing effective and targeted therapies to patients. The workshop assessed the current landscape of genomic-enabled drug discovery and development activities; examined how to enable partnerships and develop 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.

FDA commissioner Rob Califf (right) delivers the keynote presentation at the October 19 workshop on *Real-World Evidence Generation and Evaluation of Therapeutics* following remarks from workshop co-chairs (left to right) Gregory Simon and Steven Galson.

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

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 health records and personal devices. 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' 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 in October 2016, examined opportunities and challenges for 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 incorporation of real-world data throughout the product lifecycle; and use of novel approaches such as Web-based or other digital technologies. A National Academy of Medicine (NAM) Discussion Paper authored by the workshop co-chairs was made available in conjunction with the workshop.

The Drug Development Paradigm in Oncology—A Workshop (December 2016)

In collaboration with the National Cancer Policy Forum, the Forum convened a workshop in December 2016 to examine the challenges of the traditional cancer drug development process and explore opportunities to promote seamless cancer drug development. The workshop facilitated discussion about strategies to improve the collection of information across the lifecycle of cancer therapies; streamline and adapt drug development based on compelling evidence; and consider ethical and regulatory implications for a seamless cancer drug development paradigm.

Action Collaboratives:

Mapping and Connecting the Biomedical Innovation Ecosystem—Action Collaborative

The biomedical innovation ecosystem is a dynamic network of activity. Standardizing and bringing clarity to this complex process could help to set a common vocabulary and allow more fluid dialogue among ecosystem participants to encourage further innovation. It could also facilitate ongoing discussion 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. This Collaborative enabled discussion to identify rate-limiting steps in order to facilitate process improvement efforts. In 2016, Collaborative participants prepared two process maps showing the development of small molecules and biologics, which will be released to the public to aid in the identification of inputs, bottlenecks, and system gaps. Collaborative participants will author a peer-reviewed journal article and accompanying NAM Discussion Paper to inform and help guide future dialogue and progress.

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

Many argue that the current paradigm for drug discovery and development requires disruptive innovation to break out of a crisis in research and development (R&D) productivity. Evidence suggests that industries are almost always disrupted from the outside by new technologies they were slow to embrace, new business models they wrongly

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

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

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

2009

November 3
Forum Meeting #11

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

Forum member Chris Austin, middle, discusses the *Mapping and Connecting* Collaborative at the October 18th Forum meeting, alongside (left to right) invited David Altarac (Shire), Michael Rosenblatt (Merck), Richard Murray (Merck), Taylor Gilliland (NIH), and Freda Lewis-Hall (Pfizer).

dismissed, or policy changes they thought they could keep at bay. The pharmaceutical industry offers many opportunities for disruption in each of these areas. The Forum convened a Collaborative that identified and highlighted potentially breakthrough ideas and visionary approaches to the "drug development and translational science enterprise of the future." The effort addressed new technologies (e.g., biosensors, apps and telemetry, synthetic biology, new delivery technologies); new business models (e.g., crowdsourcing platforms, drug repurposing, virtual companies, clinical trials); and policy issues (e.g., pricing/reimbursement, patent law, data transparency). The effort included a data-gathering phase and review phase involving the Forum membership and culminated in the release of an NAM Discussion Paper.

April 27
Workshop: Streamlining Clinical Trial and Material Transfer Negotiations

April 27–28
Forum Meeting #12

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

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

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

October 15–16
Forum Meeting #14

Clinical Trial Site Standards Harmonization—Action Collaborative

Since sponsoring a workshop series on issues relating to the U.S. national clinical trials enterprise from 2009 to 2011, the Forum continues to devote time and attention to issues around clinical trials. This Collaborative is an ad hoc convening activity under the auspices of the Forum, which provides a venue for joint and collaborative activities among participants to advance the development of standards or a system to improve clinical trial performance through the accreditation of clinical trial sites. Participants, who are drawn from multiple sectors and disciplines, are preparing an NAM Discussion Paper that will summarize 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. Collaborative participants have also undertaken a second phase of the Collaborative activity, which includes the collection, analysis, and assessment of how clinical trial site standards that are currently in use by key stakeholders could be harmonized.

Sharing Clinical Trial Data—Action Collaborative

Sharing clinical trial data can facilitate more efficient and effective development of better medicines, diagnostics, and procedures for the ultimate benefit of patients. At the same time, sharing data presents risks, burdens, and challenges that need to be addressed by a broad set of stakeholders. These opportunities and challenges were laid out in the

2010

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

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

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

April 29–30
Forum Meeting #15

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

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 Drug 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 focusing on *Building an IT and Technical Infrastructure* and is 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 technical infrastructure. The working group convened on February 3, 2016, at the National Academies and then released its suggested way forward at a meeting at the Wellcome Trust in London in March 2016. Also launched in 2016 under the auspices of the Collaborative is a workstream addressing *Data Sharing Principles for Nonprofit Clinical Trial Funders*, which is convening nonprofit funders of research, focusing on disease advocacy and patient-focused organizations, to develop standards and policies for fostering, promoting, or requiring data sharing by grantees. This working group of the Collaborative met in July 2016 to discuss principles and strategies for nonprofit funders to share clinical trial data, including associated risks and challenges.

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

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

2011

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

March 28
Forum Meeting #18

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

Looking Forward

Forum Activities in 2017

Forum Meetings

The Forum membership will meet in March, July, and October 2017 to continue its discussions of key problems and strategies in the discovery, development, and translation of drugs. Forum workshop planning committees, working groups, and Action Collaboratives will convene activities to discuss and act on priority areas identified by them, including the following:

Workshops:

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

The Forum, in collaboration with the Roundtable on Genomics and Precision Health (the Roundtable), will convene in March 2017 a follow-up to a workshop that the two groups held in March 2016 on *Deriving Drug Discovery Value from Large-Scale Genetic Bioresources*. The 2017 workshop will examine later stages in the drug development pipeline, including the design and implementation of genetics-based clinical trials. The workshop will examine and discuss successes, challenges, and best practices for effectively utilizing 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. The workshop will consider how clinical trials with genetically identified participants can enable more efficient and effective drug development; highlight ongoing genetics-based clinical trials across a variety of diseases to examine best practices and lessons learned; learn about the logistical challenges and successes associated with genetics-based clinical trial design; and examine possible mechanisms to engage participants and improve enrollment into clinical trials based on genetic characteristics.

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

June 28–29
Forum Meeting #19

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

September 20–21
Workshop: Strengthening a Workforce for Innovative Regulatory Science in Therapeutics Development

October 4–5
Forum Meeting #20

Forum member Margaret Anderson, center, discusses the launch of the Forum Action Collaborative on the *Science of Patient Input* at the July 18 Forum meeting. (Left to right) Guest speaker Kim McCleary (*FasterCures*), and Forum members Kate Knobil, Margaret Anderson, and Brian Strom.

Examining the Impact of Real-World Evidence on Medical Product Development—A Workshop

The Forum held a workshop on October 19, 2016, titled *Real-World Evidence Generation and Evaluation of Therapeutics*, which examined opportunities for and challenges of incorporating real-world evidence into medical product development and evaluation. This workshop built on prior work of the Forum in recent years that focused on clinical trials, data sharing, and regulatory science, and will serve as the springboard for a workshop that will more fully examine the issues highlighted at the October 2016 workshop. As part of FDA's continued focus on building a national governance system for evidence generation, the focus of this workshop will be on the generation and utilization of real-world evidence to evaluate efficacy, effectiveness, tolerability, and safety for review of both new indications and postapproval studies. The workshop will provide a format for examining the practicalities of collecting real-world data and deriving and applying real-world evidence for the evaluation of medical products, including drugs, biologics, and devices. Workshop presentations and discussions will include consideration of sources of real-world data; gaps in data collection activities and priority areas that real-world evidence incorporation could address; standards and methodologies for collecting and analyzing real-world evidence in support of new indications or postapproval studies; applications for using real-world evidence to supplement traditional clinical trials; mechanisms for determining which discrete types of real-world evidence could support regulatory decisions; and operational challenges and barriers for generating and incorporating real-world evidence in the context of a learning health system.

November 7–8
Workshop: Envisioning a Transformed Clinical Trials Enterprise in the United States: Establishing an Agenda for 2020

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

2012

November 15
Russia Workshop Summary Release: *The New Profile of Drug-Resistant Tuberculosis: A Global and Local Perspective*

June 5
Forum Meeting #22

21st Century Clinical Trials—A Workshop

Clinical trials are the cornerstone of medical product development—evaluating efficacy and identifying safety issues of new drugs—and a necessary requirement for bringing new therapies to patients. The cost and duration of clinical trials have escalated dramatically in the past 20 years, greatly hampering the introduction of new therapies and driving recruitment to developing countries, raising questions about generalizability, data quality, and participant protections. Technological advances have opened the possibility for new trial designs that could increase participation in the United States, particularly through the engagement of community health centers or other local venues outside of traditional academic centers. Such trial designs could create the potential for more equitable and representative recruitment of participants as well as for more facile uptake of trial findings into clinical practice. 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 provide a venue to explore opportunities for a modern clinical trials enterprise in light of other transformative changes in the drug development and health system sectors. Participants will examine the current state of the enterprise, highlight opportunities to address barriers to further improvements, explore incentives that could foster enhanced collaboration, and discuss mechanisms for providers and health systems to take part in and encourage patient participation in clinical trials.

Action Collaboratives:

Clinical Trial Site Standards Harmonization—Action Collaborative

Collaborative participants are overseeing a second phase of the Collaborative activity, which includes the collection, analysis, and assessment of how clinical trial site standards currently in use by key stakeholders could be harmonized. In 2017, the Collaborative will release an NAM

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

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

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

October 23–24
Forum Meeting #23

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

Discussion Paper highlighting its work and a summary of the consultant-generated analysis of a sample of stakeholder standards applied to clinical trial sites.

Sharing Clinical Trial Data—Action Collaborative

In 2017, the Forum will continue to serve as a sounding board for global collaborations to advance data sharing. The Forum will also support the activities of the workstream addressing *Data Sharing Principles for Nonprofit Clinical Trial Funders* by convening a meeting of stakeholders to review principles and share experiences and information about ongoing data sharing efforts.

Science of Patient Input—Action Collaborative

Though significant successes have been realized in advancing the field of patient engagement and the science of patient input across the biomedical enterprise, there still exists a critical need to better identify priority areas for the science of patient input; identify best practices and principles for measuring patient input; advance and coalesce developed principles and taxonomies; and maintain an ongoing awareness and spirit of collaboration among the public, private, and regulatory sectors. Under the auspices of the Forum, an Action Collaborative has been convened to identify the areas of most critical need that can be best advanced through cross-sector collaboration in the neutral venue of the National Academies. In 2017, Collaborative members will meet to review, discuss, and decide on the group's specific next steps, including potential activities and outputs.

Improving the Drug Development Process—Action Collaborative

The Forum will launch, in mid-2017, an Action Collaborative that will explore avenues for improving the drug development process. The Collaborative could address, for example, the causes of late-stage failures and novel approaches that might address them.

2013

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

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

February 12
Forum Meeting #24

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

June 3
Forum Meeting #25

Forum Themes and Priorities

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

Innovation and the Drug Development Enterprise

Despite exciting scientific advances, the pathway from basic science to new therapeutics faces challenges on many fronts. New paradigms for discovering and developing drugs are being sought to bridge the ever-widening gap between scientific discoveries and translation of those discoveries into life-changing medications. There is also increasing recognition of the need for new models and methods for drug development and translational science, and "precompetitive collaborations" and other partnerships, including public–private partnerships, are proliferating. The Forum offers a venue to discuss effective collaboration in the drug discovery and development enterprise and also hosts discussions that could help chart a course through the turbulent forces of disruptive innovation in the drug discovery and development "ecosystem."

2014

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

October 28–29
Forum Meeting #26

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)

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

Science Across the Drug Development Lifecycle

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

Clinical Trials and Clinical Product Development

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

2015

June 10–11
Forum Meeting #28

January 20–21
Workshop: Financial Incentives to Support Unmet Medical Needs for Nervous System Disorders

March 26–27
Workshop: Rapid Medical Countermeasure Response to Infectious Diseases: Enabling Sustainable Capabilities Through Ongoing Public- and Private-Sector Partnerships

October 7–8
Forum Meeting #29

March 17–18
Forum Meeting #30

Infrastructure and Workforce for Drug Discovery, Development, and Translation

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

2016

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

February 3
Action Collaborative Meeting #1: IT/Technical Infrastructure for Sharing

March 22
Workshop: Deriving Drug Discovery Value from Large-Scale Genetic Bioresources

March 23
Forum Meeting #33

OVERVIEW

The Forum fosters dialogue and collaboration to illuminate and act on potentially breakthrough ideas and visionary approaches to the biomedical innovation system of the future.

July 14
Action Collaborative Meeting #1: Data Sharing Principles for Nonprofit Clinical Trial Funders

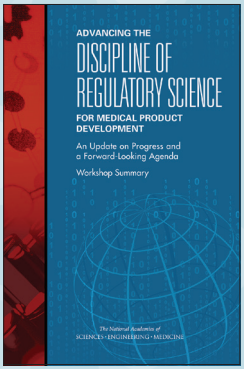
July 19–20
Forum Meeting #34

October 19
Workshop: Real-World Evidence Generation and Evaluation of Therapeutics

October 18
Forum Meeting #35

December 12–13
Workshop: The Drug Development Paradigm in Oncology

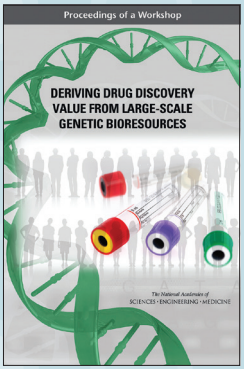
Publications Released in 2016



Advancing the Discipline of Regulatory Science for Medical Product Development: An Update on Progress and a Forward-Looking Agenda: Workshop Summary

Released: April 20, 2016

The field of endeavors known as "regulatory science" has grown out of the need to link and integrate knowledge within and among basic science research, clinical medicine, and other specific scientific disciplines whose focus, aggregation, and ultimate implementation could inform biomedical product development and regulatory decision making. On October 20–21, 2015, the Forum held a public workshop to facilitate dialogue among stakeholders about the current state and scope of regulatory sciences, opportunities to address barriers to the discipline's success, and avenues for fostering collaboration across sectors. The workshop, co-sponsored by the Burroughs Wellcome Fund, also held panels that explored key needs for strengthening the discipline of regulatory science, including considering what are the core components of regulatory science infrastructure to foster innovation in medical product development. To focus the workshop discussions, the planning committee adopted the theme of innovation in regulatory science through integration of information. This publication is a summary of the workshop.

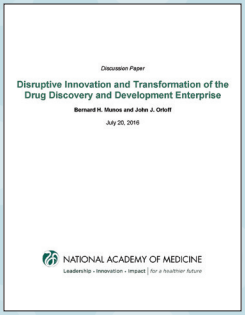


Deriving Drug Discovery Value from Large-Scale Genetic Bioresources: Proceedings of a Workshop (collaboration with the Roundtable on Genomics and Precision Health)

Released: September 9, 2016

Utilizing genetic and phenotypic information collected and stored in bioresources has the potential to enable more efficient drug discovery and precision medicine. Within this context, the Roundtable on Genomics and Precision Health (previously called the Roundtable on Translating Genomic-Based Research for Health) and the Forum hosted a workshop on March 22, 2016, titled *Deriving Drug Discovery Value from Large-Scale Genetic Bioresources*. The workshop focused on new research and ideas in three primary areas: large-scale genetic cohort studies, the use of genomic data in drug discovery activities, and novel business models that support the development and use of genetic data from bioresources for drug discovery. Throughout the workshop there was robust discussion of short-term and long-term opportunities for collaboration, fostering translational research, and accelerating progress in the area of genomic-enabled drug discovery. This publication summarizes the presentations and discussions held at the workshop.

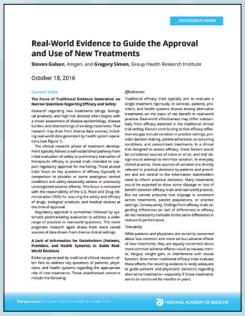
Related NAM Perspectives



Disruptive Innovation and Transformation of the Drug Discovery and Development Enterprise (By Bernard H. Munos and John J. Orloff)

July 20, 2016

Declining or stagnant R&D productivity has led many observers to argue that the current paradigm for drug discovery and development requires disruptive innovation to break out of this crisis by identifying and rapidly bringing new discoveries to market. Despite increased investment, the number of new therapies and improvements to human health as measured by the growth in life expectancy have remained relatively constant over the past 50 to 60 years. Sustained competition from generic manufacturers and overall negative public reactions to costly prescription drugs only add to the complex challenges facing large pharmaceutical companies today. Critics cite the need for the industry to produce more and better products and affordably innovate if it hopes to survive. To further assess the challenges and reveal potential opportunities, participants of an Action Collaborative convened under the auspices of the Forum engaged thought leaders and key stakeholders within the biomedical research ecosystem, soliciting diverse viewpoints to gain insight into their unique perspectives on the state of the pharmaceutical and biomedical research industries, what could or should change, how those changes might occur, and, generally, what the future might hold.



Real-World Evidence to Guide the Approval and Use of New Treatments (By Steven Galson and Gregory Simon)

October 18, 2016

Research regarding new treatments (drugs, biological products, and high-risk devices) often begins with a broad assessment of disease epidemiology, disease burden, and the shortcomings of existing treatments. That research may draw from diverse data sources, including real-world data generated by health system operations. The clinical research phase of treatment development typically follows a well-established pathway from initial evaluation of safety to preliminary evaluation of therapeutic efficacy to pivotal trials intended to support regulatory approval for marketing. This trajectory is consistent with the responsibility of FDA for ensuring the safety and efficacy of drugs, biological products, and medical devices at the time of approval. However, the traditional process for evaluating new therapeutics does not produce the evidence that patients, clinicians, and payers need for real-world decisions. This Discussion Paper reviews the current state of real-world evidence generation, how stakeholders can best enable developments for producing high-quality, real-world evidence, case studies highlighting promising practices for the generation and utilization of real-world evidence, and a vision for the desired future state of real-world evidence generation for medical product development.

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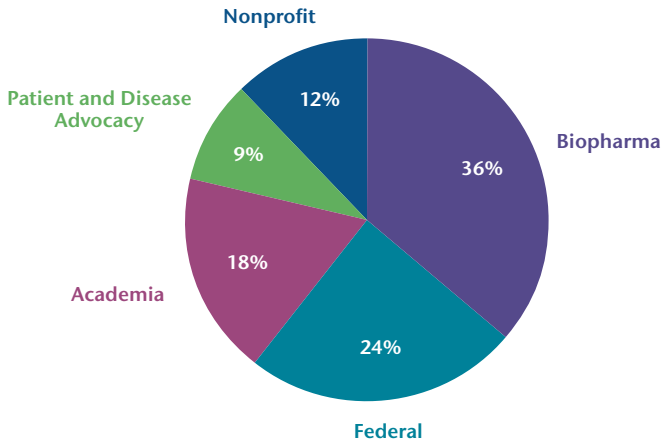

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The Forum on Drug Discovery, Development, and Translation of the National Academies of Sciences, Engineering, and Medicine was created in 2005 by the National Academies' Board on Health Sciences Policy to provide a unique platform for dialogue and 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 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.

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