The National Academies of SCIENCES ENGINEERING MEDICINE

Forum on DRUG DISCOVERY, DEVELOPMENT, and TRANSLATION

Envisioning a Transformed Clinical Trials Enterprise for 2030

A Four-Part Virtual Workshop

Part 1: January 26, 2021

Table of Contents

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A Four-Part Virtual Workshop

January 26, February 9, March 24, and May 11, 2021

Clinical trials research has changed dramatically over the last decade. The biological, physical, and digital spheres are merging; clinical research and health care are at a critical juncture; new approaches enable the collection of data in real-world settings; and new modalities, such as digital health technologies and artificial intelligence applications, are changing possibilities for the conduct of clinical research. 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 is strained 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 National Academies 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 to consider lessons learned from progress and setbacks over the past 10 years and broadly consider goals and key priorities for advancing a clinical trials enterprise that is more efficient, effective, person-centered, inclusive, and integrated into the health delivery system of 2030.

A planning committee of the National Academies of Sciences, Engineering, and Medicine will plan and conduct a virtual public workshop designed to consider a transformed clinical trial enterprise for 2030, featuring invited presentations and discussions on:

- Lessons learned from progress and setbacks over the past 10 years.
- How an envisioned 2030 clinical trials enterprise might differ from the current system.
- The following core themes in framing a 2030 agenda:
    - Diversity and inclusion of clinical trial participants
    - Convergence of clinical research and clinical practice
    - Clinical trial data sharing
    - Incorporation of new technologies into drug research and development
    - Workforce and career development
    - Public engagement and partnership
    - Regulatory Environment
    - Cultural and Financial Incentives
- Key priority challenges and opportunities when it comes to the 2030 clinical trials enterprise.
- Practical short- and long-term goals for improving the efficiency, effectiveness, person-centeredness, inclusivity, and integration with healthcare of the clinical trials enterprise.

The planning committee will organize the workshop, develop the agenda, select and invite speakers and discussants, and moderate or identify moderators for the discussions. A proceedings of the presentations and discussions at the workshop will be prepared by a designated rapporteur in accordance with institutional guidelines.

Planning Committee

Steven Galson (co-chair), Amgen

Esther Krofah (co-chair), FasterCures, Milken Institute

Amy Abernethy, Office of the Commissioner, FDA

Anita LaFrance Allen, University of Pennsylvania

Christopher P. Austin, National Center for Advancing Translational Sciences, NIH

Howard A. Burris III, Sarah Cannon

Luther T. Clark, Merck & Co., Inc.

Giselle Corbie-Smith, The University of North Carolina at Chapel Hill

M. Khair ElZarrad, Center for Drug Evaluation and Research, FDA

Jennifer Goldsack, Digital Medicine Society

Richard A. Moscicki, PhRMA

Amy Patterson, National Heart, Lung, and Blood Institute, NIH

Joseph Scheeren, Critical Path Institute

Anantha Shekhar, University of Pittsburgh

Pamela Tenaerts, Clinical Trials Transformation Initiative

Christopher Yoo, Systems Oncology

Envisioning a Transformed Clinical Trials Enterprise for 2030

A Four-Part Virtual Workshop

Part 1: January 26, 2021

REGISTRATION: https://www.eventbrite.com/e/envisioning-a-transformed-clinical-trials-enterprise-for-2030-a-workshop-tickets-127794608113

WATCH THE WEBCAST: https://www.nationalacademies.org/event/10-28-2020/envisioning-a-transformed-clinical-trials-enterprise-for-2030-a-workshop

This virtual public workshop will provide a venue for stakeholders to consider a transformed clinical trial enterprise for 2030. Workshop participants will consider lessons learned from progress and setbacks over the past 10 years, since the previous workshop, Envisioning a Transformed Clinical Trials Enterprise in the United States, and, looking forward, discuss goals and key priorities for advancing a clinical trials enterprise that is more efficient, effective, person-centered, inclusive, and integrated into the health delivery system of 2030.

This virtual workshop will be conducted in four parts:

- **Part One (January 26, 2021) will provide an overview discussion on how an envisioned 2030 clinical trials enterprise may differ from the current system. It will discuss key challenges and opportunities in improving person-centeredness and inclusivity, building resilience and transparency, and integrating new technologies. Specifically, workshop discussions will**
    - **Highlight lessons learned from progress and setbacks over the past 10 years**
        - **Consider components of the clinical trials enterprise that have been modified or abandoned in response to COVID-19 that establish positive and sustainable examples of change**
    - **Consider how an envisioned 2030 clinical trials enterprise might differ from the current system**
- Part Two (February 9, 2021) will consider achievable goals to enhance person-centeredness and inclusivity in the clinical trials enterprise; and discuss ways to improve public engagement and partnership.
- Part Three (March 24, 2021) will consider approaches to build resilience, sustainability, and transparency. The discussion will include the convergence and integration of clinical research and clinical practice; data sharing and management; and efficient, engaging scientific communication.
- Part Four (May 11, 2021) will consider ways the thoughtful and deliberate use of new technologies could improve the clinical trials enterprise and support goals outline in prior webinar sessions.

For additional information on the virtual workshop, please visit the main project page.

Workshop Part 1: January 26, 2021

11:00 AM – 3:30 PM ET

11:00 a.m. **Welcome and Opening Remarks**
STEVEN GALSON, *Workshop Co-chair*
Senior Vice President, Global Regulatory Affairs and Safety
Amgen, Inc.

ESTHER KROFAH, *Workshop Co-chair*
Executive Director
FasterCures, Milken Institute

SESSION I: A MORE PERSON-CENTERED AND INCLUSIVE CLINICAL TRIALS ENTERPRISE

Session Objective

- Discuss key priority challenges and opportunities when it comes to person-centeredness and inclusivity in the 2030 clinical trials enterprise

11:10 a.m. **A Story in Action: Person-Centeredness and Inclusivity**
TERRIS KING
Former Director, Office of Minority Health
Centers for Medicare and Medicaid Services

11:25 a.m. **Facilitated Breakout Groups** *(30mins)*

Discussion Questions:

- *Do you agree with the proposed goals listed below for enhancing person-centeredness and inclusivity?*
- *What would you change, and how?*
- *What are potential interim actions or milestones that might be key to achieving these goals?*

Goals to consider for enhancing person-centeredness and inclusivity

- Improve representation and relevance
- Improve community engagement, transparency, and "user-friendliness" to foster trust, counter misinformation, and meet the needs of patients
- Demonstrate trustworthiness to the general public of clinical trials
- Engage and prepare a diverse clinical research workforce

11:55 a.m. **Breakout Group Report-outs** *(10mins)*

12:10 p.m. **BREAK** *(30mins)*

SESSION II: A MORE RESILIENT, SUSTAINABLE, AND TRANSPARENT CLINICAL TRIALS ENTERPRISE

Session Objectives

- Discuss key priority challenges and opportunities when it comes to building a more resilient, sustainable, and transparent clinical trials enterprise

12:45 p.m. **The State of Clinical Trials in 2021: A Perspective from Industry**
ELLIOTT LEVY
Sr. Vice President, R&D Strategy and Operations
Amgen, Inc.

1:00 p.m. **A Story in Action: Building a More Resilient, Sustainable, and Transparent Clinical Trials Enterprise**
JANET WOODCOCK
Acting Commissioner of Food and Drugs
U.S. Food and Drug Administration

1:15 p.m. **Facilitated Breakout Groups** *(30mins)*

Discussion Questions:

- *Do you agree with the straw vision statement for building a more resilient, sustainable, and transparent clinical trials enterprise (below)?*
- *What would you change, and how?*
- *What are some potential interim actions or milestones that might be key to achieving these goals?*

Goals to consider for building a more resilient, sustainable, and transparent clinical trials enterprise

- Improve community engagement, transparency, and "user-friendliness" to foster trust, counter misinformation, and meet the needs of patients
- Reduce complexity and streamline trials and trial start-up, and standardize key data elements
- Support regulatory robustness, flexibility, and built-in ability to adjust (e.g., in times of stress, to handle new tech robustly)
- Reduce conduction of "uninformative" clinical trials and prioritize resources to robustly-designed trials
- Generate a larger amount of high-quality evidence at lower cost
- Reduce risk aversion to improve research questions and trial design innovation
- Embrace novel statistical techniques to power trials
- Connect and embed clinical care and clinical research

1:45 p.m. **Breakout Group Report-outs** *(10mins)*

2:00 p.m. **BREAK** *(30mins)*

SESSION III: MORE APPROPRIATE USE OF TECHNOLOGIES TO OPTIMIZE THE CLINICAL TRIALS ENTERPRISE

Session Objective

- Discuss key priority challenges and opportunities when it comes to appropriately using new technologies to optimize the 2030 clinical trials enterprise

2:30 p.m. **A Story in Action: Optimizing with New Technologies**
ROBERT CALIFF
Head of Clinical Policy and Strategy
Verily Life Sciences and Google Health

2:45 p.m. **Facilitated Breakout Groups** *(30mins)*

Discussion Questions:

- *Do you agree with the proposed goals listed below for more appropriately using new technologies to optimize the clinical trials enterprise?*
- *What would you change, and how?*
- *What are some potential interim actions or milestones that might be key to achieving these goals?*

Goals to consider for more appropriately using technology to optimize the clinical trials enterprise

- Decentralize clinical trials
- Use digital tools for clinical trials management
  - Develop resources to help institutions that need more support
- Increase local capacity for research innovation
- Collate efforts to frame new technologies as part of an ecosystem rather than a series of unrelated one-off tech solutions
- Develop and deploy systems and tools to combine many sources of data
- Incorporate patient input into research
- Advance analytics for recruitment and analysis

3:15 p.m. **Breakout Group Report-outs** *(10mins)*

WRAP UP

3:30 p.m. **Wrap Up Discussion and Closing Remarks**
STEVEN GALSON, *Workshop Co-chair*
Senior Vice President, Global Regulatory Affairs and Safety
Amgen, Inc.

ESTHER KROFAH, *Workshop Co-chair*

Executive Director
FasterCures, Milken Institute

3:35 p.m. **Adjourn**

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Planning Committee Biographies

CO-CHAIRS

STEVEN K. GALSON (co-chair), M.D. is the senior vice president, Global Regulatory Affairs and Safety at Amgen. He joined Amgen in 2010 as vice president, Global Regulatory Affairs. Prior to Amgen, Dr. Galson was senior vice president for Civilian Health Operations and chief health scientist at Science Applications International Corporation. Dr. Galson spent more than 20 years in government service, including two years as acting Surgeon General of the United States. Previously, he served as director of the Food and Drug Administration's (FDA) Center for Drug Evaluation and Research (CDER), where he provided leadership for the center's broad national and international programs in pharmaceutical regulation. Dr. Galson began his Public Health Service (PHS) career as an epidemiological investigator at the Centers for Disease Control and Prevention (CDC) after completing a residency in internal medicine at the Hospitals of the Medical College of Pennsylvania. He also held senior-level positions at the Environmental Protection Agency (EPA); the Department of Energy, where he was chief medical officer; and the Department of Health and Human Services. Prior to his arrival at the FDA, Galson was director of the EPA's Office of Science Coordination and Policy, Office of Prevention, Pesticides and Toxic Substances. He holds a B.S. from Stony Brook University, an M.D. from Mt. Sinai School of Medicine, and an M.P.H. from the Harvard School of Public Health.

ESTHER KROFAH (co-chair), M.P.P., is the executive director of FasterCures, a center of the Milken Institute. She has deep experience in the government, nonprofit, and for-profit sectors, where she has led efforts to bring together diverse stakeholder groups to solve critical issues and achieve shared goals that improve the lives of patients. Most recently, Ms. Krofah was the director of public policy leading GlaxoSmithKline's engagement with the U.S. Department of Health and Human Services (HHS) and relevant Executive Branch agencies on broad health-care policy issues, including leadership in improving vaccinations and care for people living with HIV. Prior to GSK, Ms. Krofah served as the deputy director of HHS' Office of Health Reform, where she led the development of policy positions for significant regulator priorities, including the health insurance marketplaces. Prior to HHS, Ms. Krofah served as a program director at the National Governors Association (NGA) health-care division, working directly with governors' health policy advisors, state Medicaid directors, and state health commissioners on health insurance, health workforce, and Medicaid coverage issues. Before joining the NGA, Ms. Krofah worked in consulting at Deloitte Consulting LLP, where she worked with public sector and commercial clients, including assisting states in developing state-based exchanges. Ms. Krofah received a B.A. from Duke University and a Masters of Public Policy from the Harvard University John F. Kennedy School of Government.

MEMBERS

AMY ABERNETHY, M.D., Ph.D, is an oncologist and internationally recognized clinical data expert and clinical researcher. As the Principal Deputy Commissioner of Food and Drugs, Dr. Abernethy helps oversee FDA's day-to-day functioning and directs special and high-priority cross-cutting initiatives that impact the regulation of drugs, medical devices, tobacco and food. As acting Chief Information Officer, she oversees FDA's data and technical vision, and its execution. She has held multiple executive roles at Flatiron Health and was professor of medicine at Duke University School of Medicine, where she ran the Center for Learning Health Care and the Duke Cancer Care Research Program. Dr. Abernethy received her M.D. at Duke University, where she did her internal medicine residency, served as chief resident, and completed her hematology/oncology fellowship. She received her Ph.D. from Flinders University, her B.A. from the University of Pennsylvania and is boarded in palliative medicine.

ANITA LAFRANCE ALLEN, J.D., PH.D., is an internationally renowned expert on privacy law and ethics, and is recognized for contributions to legal philosophy, women's rights, and diversity in higher education. In July 2013, Dr. Allen was appointed Penn's Vice Provost for Faculty, and in 2015, Chair of the Penn Provost's Advisory Council on Arts, Culture and the Humanities. From 2010 to 2017, she served on President Obama's Presidential Commission for the Study of Bioethical Issues. She was presented the Lifetime Achievement Award of the Electronic Privacy Information Center in 2015 and elected to the National Academy of Medicine in 2016.

In 2017 Dr. Allen was elected Vice-President/President Elect of the Eastern Division of the American Philosophical Association. In 2015, Dr. Allen was on the summer faculty of the School of Criticism and Theory at Cornell. A two-year term as an Associate of the Johns Hopkins Humanities Center concluded in 2018. Her books include *Unpopular Privacy: What Must We Hide* (Oxford, 2011); *Privacy Law and Society* (Thomson/West, 2017); *The New Ethics: A Guided Tour of the 21st Century Moral Landscape* (Miramax/Hyperion, 2004); and *Why Privacy Isn't Everything: Feminist Reflections on Personal Accountability* (Rowman and Littlefield, 2003).

CHRISTOPHER P. AUSTIN, M.D., is Director of the National Center for Advancing Translational Sciences (NCATS) at the U.S. National Institutes of Health. NCATS' mission is to catalyze the generation of innovative methods and technologies that will enhance the development, testing, and implementation of diagnostics and therapeutics across a wide range of human diseases and conditions. Before becoming NCATS Director in September 2012, he was director of the NCATS Division of Preclinical Innovation, which focuses on translating basic science discoveries into new treatments, particularly for rare and neglected diseases, and developing new technologies and paradigms to improve the efficiency of therapeutic and diagnostic development. In this role, he founded and directed numerous initiatives including the NIH Chemical Genomics Center (NCGC), the Therapeutics for Rare and Neglected Diseases (TRND) program, and the Toxicology in the 21st Century (Tox21) program. Before joining NIH in 2002, Dr. Austin directed research programs genomics-based target discovery, pharmacogenomics, and neuropsychiatric drug development at Merck, with a particular focus on schizophrenia. Dr. Austin received his A.B. in biology from Princeton and M.D. from Harvard Medical School. He completed clinical training in internal medicine and neurology at the Massachusetts General Hospital, and a fellowship in genetics at Harvard.

HOWARD A. BURRIS III, M.D., serves as president and chief medical officer of Sarah Cannon, as well as the executive director, drug development for the research institute. He is an associate of Tennessee Oncology, PLLC, where he practices medical oncology. Dr. Burris' clinical research career has focused on the development of new cancer agents with an emphasis on first in human therapies, having led the trials of many novel antibodies, small molecules, and chemotherapies now FDA approved, including ado-trastuzumab emtansine, everolimus, and gemcitabine. In 1997, he established in Nashville the first community based early phase drug development program, which grew into the Sarah Cannon Research Institute. He has authored over 400 publications and 700 abstracts. Sarah Cannon has now dosed over 350 first in human anticancer therapies and enrolls more than 3000 patients per year into clinical trials. Dr Burris will serve as the elected president of ASCO in 2019-2020. He also currently serves on the Board of ASCO's Conquer Cancer Foundation. Additionally in 2014, Dr. Burris was selected by his peers as a Giant of Cancer Care for his achievements in drug development.

Dr. Burris completed his undergraduate education at the United States Military Academy at West Point, his medical degree at the University of South Alabama, and his internal medicine residency and oncology fellowship at Brooke Army Medical Center in San Antonio. While in Texas, he also served as the Director of Clinical Research at The Institute for Drug Development of the Cancer Therapy and Research Center and The University of Texas Health Science Center. He attained the rank of lieutenant colonel in the US Army, and among his decorations, he was awarded a Meritorious Service Medal with oak leaf cluster for his service in Operation Joint Endeavor.

LUTHER T. CLARK, M.D, is Deputy Chief Patient Officer and Global Director, Scientific Medical and Patient Perspective in the Office of the Chief Patient Officer at Merck. In this role, he is responsible for (1) gathering internal and external scientific and medical information to assist with decision-making at the highest levels; (2) collaborating across Merck to increase the voice of patients, directly and indirectly in decision-making; (3) collaborating with key internal and external stakeholders in development of a systematized approach for collecting and incorporating patient insights across the patient journey and product lifecycle; and (4) representing Merck externally, expanding bi-directional exchange with key patient and professional leaders and organizations.

Dr. Clark leads Merck's Patient Insights Team, is co-leader of the team that champions Health Care Equities (including promotion of health literacy and research diversity) and chairs the Patient Engagement, Health Literacy & Clinical Trials Diversity Investigator Initiated Studies Research Committee.

Prior to joining Merck, Dr. Clark was Chief of the Division of Cardiovascular Medicine at the State University of New York Downstate Medical Center (SUNY Downstate) and founding Director of the NIH-funded Brooklyn Health Disparities Research Center. Dr. Clark earned his Bachelor of Arts degree from Harvard College and his Medical degree from Harvard Medical School. He is a Fellow of the American College of Cardiology and the American College of Physicians, and a past member of the Board of Directors of the Founders Affiliate of the American Heart Association. He is a nationally and internationally recognized leader in cardiovascular education, clinical investigation, cardiovascular disease prevention, and health equity. He has authored more than 100 publications and edited and was principal contributor to the textbook Cardiovascular Disease and Diabetes (McGraw-Hill).

Dr. Clark has received numerous awards and honors, including the Harvard University Alumni Lifetime Achievement Award for Excellence in Medicine. He is the current President of the Health Science Center at Brooklyn Foundation, SUNY Downstate Medical Center.

GISELLE CORBIE-SMITH, M.D., M.Sc., is nationally recognized for her scholarly work on the inclusion of disparity populations in research, and has over a decade of experience in using community engagement to conduct innovative, translational health equity research. Her empirical work, using both qualitative and quantitative methodologies, has focused on the methodological, ethical, and practical issues of research to address racial disparities in health. A Kenan Distinguished Professor in the Departments of Social Medicine and Medicine at the UNC School of Medicine in Chapel Hill, NC, Dr. Corbie-Smith has served as the Principal Investigator of several community-based participatory research projects focused on disease risk reduction among rural racial and ethnic minorities. These projects have included funding through the National Heart Lung and Blood Institute, the Robert Wood Johnson Foundation, the National Center for Minority Health and Health Disparities, the National Institute of Nursing Research, Greenwall Foundation, and the National Human Genome Research Institute.

Dr. Corbie-Smith is accomplished in drawing communities, faculty, and health care providers into working partnerships in clinical and translational research. This engagement ultimately transforms the way that academic investigators and community members interact while boosting public trust in research. She has also shown a deep commitment to working in North Carolina by bringing research to communities, involving community members as partners in research, and improving health of minority populations and underserved areas.

In 2013, she established and became Director of the UNC Center for Health Equity Research to bring together collaborative multidisciplinary teams of scholars, trainees, and community members to improve North Carolina

communities' health through shared commitment to innovation, collaboration, and health equity. Dr. Corbie-Smith is currently the Co-PI for RWJF's Advancing Change Leadership Clinical Scholars Program, which provides intensive learning, collaboration, networking, and leadership development to seasoned clinicians to create a community of practitioners promoting health equity across the country. She recently served as the President of the Society of General Internal Medicine. In 2018, she was elected to the National Academy of Medicine.

M. KHAIR ELZARRAD, PH.D., M.P.H., is the Deputy Director of the Office of Medical Policy (OMP) at FDA's Center for Drug Evaluation and Research (CDER), where he leads the development, coordination, and implementation of medical policy programs and strategic initiatives. Dr. ElZarrad currently leads multiple projects focused on exploring the potential utility of real-world evidence, innovative clinical trial designs, and the integration of technological advances in pharmaceutical development. Dr. ElZarrad is the rapporteur for the International Council for Harmonisation's ongoing work to revise the international Good Clinical Practice Guideline (ICH-E6(R2)). Prior to joining the FDA, he served as Acting Director of the Clinical and Healthcare Research Policy Division with the Office of Science Policy at the National Institutes of Health (NIH). At NIH, he worked on policies related to human subject protections; the design, conduct, and oversight of clinical research; and enhancing quality assurance programs at pharmaceutical development and production facilities. He earned a doctoral degree in medical sciences with a focus on cancer metastases from the University of South Alabama, as well as a master's degree in public health from the Johns Hopkins Bloomberg School of Public Health.

JENNIFER GOLDSACK, M.A., M.B.A., is the Executive Director at the Digital Medicine Society (DiME), a new professional organization promoting the adoption of digital technologies for health. Previously, Ms. Goldsack spent several years at the Clinical Trials Transformation Initiative (CTTI) where she led development and implementation of several projects within CTTI's Mobile Program and was the operational co-lead on the first randomized clinical trial using FDA's Sentinel System. Ms. Goldsack spent five years working in research at the Hospital of the University of Pennsylvania, first in Outcomes Research in the Department of Surgery and later in the Department of Medicine. More recently, Ms. Goldsack helped launch the Value Institute, a pragmatic research and innovation center embedded in a large academic medical center in Delaware. Ms. Goldsack earned her master's degree in chemistry from the University of Oxford, England, her masters in the history and sociology of medicine from the University of Pennsylvania, and her MBA from the George Washington University. Additionally, she is a certified Lean Six Sigma Green Belt and a Certified Professional in Healthcare Quality. Ms Goldsack is a retired athlete, formerly a Pan American Games Champion, Olympian and World Championship silver medalist.

RICHARD A. MOSCICKI, M.D., is the executive vice president of science and regulatory advocacy and the chief medical officer at the Pharmaceutical Research and Manufacturers of America (PhRMA). Dr. Moscicki came to PhRMA in 2017 after serving as the deputy center director for science operations for FDA's Center for Drug Evaluation and Research (CDER) since 2013. While at FDA, Dr. Moscicki brought executive direction of Center operations and leadership in overseeing the development, implementation, and direction of CDER's programs. Previous positions include serving as chief medical officer at Genzyme Corporation from 1992 to 2011 where he was responsible for worldwide global regulatory and pharmacovigilance matters, as well as all aspects of clinical research and medical affairs for the company. He served as a senior vice president and head of clinical development at Sanofi-Genzyme from 2011-2013.

Dr. Moscicki received his medical degree from Northwestern University Medical School. He is board certified in internal medicine, diagnostic and laboratory immunology, and allergy and immunology. He completed his residency in Internal Medicine, followed by a fellowship at Massachusetts General Hospital (MGH) in clinical immunology and immunopathology. He remained on staff at MGH and on the faculty of Harvard Medical School from 1979 until 2013.

AMY PATTERSON, M.D., the Chief Science Advisor and Director of Scientific Research Programs, Policy, and Strategic Initiatives in the Immediate Office of the Director (IOD) of the National Heart, Lung, and Blood Institute (NHLBI), part of the National Institutes of Health (NIH). In this role, she provides leadership and strategic coordination of trans-NHLBI efforts and manages a broad portfolio of issues germane to the conduct of clinical

research, research oversight, policy development, major new scientific initiatives, and relationships with organizations within and external to the Institute.

Prior to joining the NHLBI in 2015, Dr. Patterson served as the NIH Associate Director for Science Policy and as the NIH Associate Director for Biosecurity and Biosafety Policy. Her responsibilities encompassed areas such as human subjects protections; the organization and oversight of clinical trials; scientific, social, and ethical considerations in genetics research and human gene transfer trials; and safety and security implications of emerging new technologies.

Prior to coming to the NIH Office of the Director, she served as the Deputy Director of the Division of Cellular and Gene Therapies and Medical Officer in the Division of Clinical Trial Design and Analysis at the FDA Center for Biologics Evaluation and Research. Dr. Patterson received her B.A. (Cum Laude) in biology from Harvard University and her M.D. (Alpha Omega Alpha) from Albert Einstein College of Medicine. She conducted her internship and residency in internal medicine at New York Hospital and Memorial Sloan Kettering and completed her post-doctoral clinical research fellowships in adult and pediatric endocrinology and metabolism at the NIH.

JOSEPH SCHEEREN, PharmD, started his pharmaceutical industry career in 1982 with Servier in Paris, responsible for Regulatory Affairs Northern and Eastern Europe, and Clinical Development in Munich from 1986 - 1987. In 1991, he was appointed Head of Worldwide Regulatory Affairs at Serono, Geneva. In 1992, he took over responsibility of the Global Regulatory Affairs department of Roussel UCLAF in Paris. In 1996, he moved to New Jersey to head the Global Marketed Product Regulatory Affairs Department of Hoechst Marion Roussel. After the merger with Rhone Poulenc Rorer in 2000, he was nominated to a similar position. Dr. Scheeren joined Bayer Pharmaceuticals as Senior Vice President, Head of Global Regulatory Affairs (GRA), in 2004, responsible for development in the US and in 2009 became Site Head US in Montville, NJ. In 2012, he assumed in addition to his responsibilities as Head of GRA, the position of Head of Global Development Asia in Beijing and in 2015, was appointed Head of GRA Pharma and Consumer Care of Bayer Healthcare, Basel. In January 2018, he was appointed Senior Advisor R&D, Bayer AG in Berlin and left Bayer AG at the end of 2018. Since January 2019, he has been Adjunct Professor at Peking University for Regulatory Sciences in the Department of Clinical Research. He became President & CEO of Critical Path Institute in April of 2019, headquartered in Tucson, Arizona. Dr. Scheeren holds many memberships and designations, serving on Advisory Boards at the Center for Innovation in Regulatory Science, the Regulatory Affairs track at Yale University, and the Center of Regulatory Excellence in Singapore. He is also a foreign member of the Academie Nationale de Pharmacie, France, and a lecturer at Yale University. Dr. Scheeren studied pharmacy at the University of Leiden.

ANANTHA SHEKHAR, M.D., PH.D., is a nationally recognized educator, researcher, and entrepreneaur with major contributions in medicine and life sciences. He was recently named senior vice chancellor for health sciences and the John and Gertrude Petersen Dean of the School of Medicine at the University of Pittsburgh. Dr. Shekhar previously lead the Indiana University School of Medicine's research enterprise and held several leadership posts at IU and IU Health. Dr. Shekhar was named executive associate dean for research affairs in August 2015, overseeing all research-related activities at the IU School of Medicine. He is one of six executive associate deans who make up the school's executive leadership team with Dean Jay L. Hess, MD, PhD. Dr. Shekhar is the founding director of the Indiana Clinical and Translational Sciences Institute (Indiana CTSI), a statewide institute within Indiana University School of Medicine, supported by a CTSA grant from the US National Institutes of Health and established in 2008 as a joint partnership of Indiana University, Purdue University, the University of Notre Dame and numerous life sciences businesses and community organizations. The Institute's mission is to assist in the rapid translation of new discoveries into novel treatments. In addition to his roles with the Indiana CTSI and IU School of Medicine, Dr. Shekhar is the Associate Vice President for University Affairs, Indiana University; Executive Vice President of Academic Affairs for Clinical Research, IU Health; August M. Watanabe Professor of Medical Research, Professor of Psychiatry, Neurobiology and Pharmacology & Toxicology at IU School of Medicine.

PAMELA TENAERTS, M.D., M.B.A., is Executive Director of CTTI. Dr. Tenaerts works closely with the CTTI Executive Committee to develop and implement strategies to accomplish CTTI's mission. She provides senior level oversight of the day-to-day operations of CTTI and orchestrates efforts to effectively engage all interested stakeholders to improve the conduct of clinical trials. She is a member of PCORI's CTAP expert post-award subcommittee and MIT's

Collaborative Initiatives Clinical Trials Process Expert Advisory Board, and a Member of the Advisory Council North America, DIA. With more than 20 years' experience in the conduct of clinical trials across a number of sectors, she practiced medicine in both the emergency department and private practice setting for several years before embarking on a career in research. Most recently, Dr. Tenaerts oversaw European operations for CoAxia, a medical device company focused on cerebral ischemia. She received her MD from Catholic University of Leuven, Belgium, and a MBA from the University of South Florida. She speaks five languages and has obtained Six Sigma Green Belt certification.

CHRISTOPHER YOO, PH.D., has over 25 years of experience in advancing cutting-edge biomedical information technology. He is the founder and CEO of Systems Imagination, Inc., which offers advanced cognitive computing technology to translate big data into valuable insights. At IBM, he was instrumental as Head of Strategy and Planning for IBM's Information Based Medicine business, a new unit that pioneered the infusion of high performance computing and research into the nascent fields of personalized medicine and molecular therapeutics based on genomics. Throughout his career, he has also held leadership positions at Cisco, Oracle, and Applied Biosystems.

As a serial entrepreneur, Dr. Yoo has created value in new companies that accelerate the adoption of smarter, technology-based systems such as cognitive computing, big data analytics, and knowledge engineering. He has founded and successfully engineered the exit of startups including MedTrust Online, the world's first and largest online community of cancer doctors treating difficult cases with molecular medicine knowledge. He has also founded Golden Gateway Partners, a trans-Pacific technology consulting company, TransMed Partners, the first boutique consultancy for translational medicine, and LabBook, the first electronic laboratory notebook for HCLS researchers.

In addition to his leadership roles at Systems Oncology and Systems Imagination, Dr. Yoo is also a Faculty Associate in the College of Health Solutions at Arizona State University. Dr. Yoo received his PhD in Cell and Molecular Biology from Yale University, and completed his postdoctoral fellowship at UC Berkeley.

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Speaker Biographies

SPEAKERS

ROBERT CALIFF, M.D., MACC, is the Head of Clinical Policy and Strategy for Verily Life Sciences and Google Health for Verily and Google Health. Prior to this Dr. Califf was the vice chancellor for health data science for the Duke University School of Medicine; director of Duke Forge, Duke's center for health data science; and the Donald F. Fortin, MD, Professor of Cardiology. He served as Deputy Commissioner for Medical Products and Tobacco in the U.S. Food and Drug Administration (FDA) from 2015-2016, and as Commissioner of Food and Drugs from 2016-2017. A nationally and internationally recognized leader in cardiovascular medicine, health outcomes research, healthcare quality, and clinical research, Dr. Califf is a graduate of Duke University School of Medicine. Dr. Califf was the founding director of the Duke Clinical Research Institute and is one of the most frequently cited authors in biomedical science.

TERRIS KING, SC.D., is the Senior Pastor of Liberty Grace Church of God, the Executive Director of the Grace Foundation and a retired federal government Senior Executive. He is a second generation ordained Baptist preacher of a growing and vibrant ministry.

He is the former Director and Client Executive, of AT&T. He was the lead executive for all AT&T services within the Department of Health and Human Services. These services exceeded $50 million.

He previously served as the Deputy Director of the Office of Information Systems at the Centers for Medicare & Medicaid Services (CMS). The Innovation initiatives Terris is establishing are the Centers future payment and health care coordination models. Prior this role, Terris was the Founder of the Office of Minority Health for the Centers of Medicare & Medicaid Services (CMS). In this role, Terris focused on the establishment of the new office with the mission to improve the health of racial and ethnic minority populations. For six years, Terris served as Deputy Director of the Office of Clinical Standards and Quality (OCSQ). The Office of Clinical Standards and Quality, (OCSQ) CMS is a division of the United States Department of Health and Human Services.

Terris is a proud alumnus of Walbrook Senior High School in Baltimore, Maryland from which he graduated and received a scholarship in football.

He completed both his Scientific Doctorate in Occupational Science and his undergraduate degree in Mass Communications from Towson University. He also completed his Masters in Behavioral Science from Johns Hopkins University and completed a Harvard University Executive Fellow. He received his Doctorate in Divinity from Saint Thomas College.

Terris is married to Catherine and has two adult children: Terris II and Miya.

ELLIOTT LEVY, M.D., is senior vice president, R&D Strategy and Operations, responsible for delivering the operational and transformational capabilities essential to executing Amgen's R&D strategy. Levy joined Amgen in 2014 and was senior vice president, Global Development, responsible for the clinical development of Amgen's pipeline.

Before joining Amgen, Levy served as senior vice president and head of Specialty Development at Bristol-Myers Squibb (BMS). Prior to that role, he held the position of senior vice president of Global Pharmacovigilance and Epidemiology. Levy joined BMS in 1997 and during his 17 years at the company, he held a range of senior positions in cardiovascular clinical development, immunoscience clinical research, and global clinical research operations.

Prior to BMS, Levy was a member of the Renal Division at Brigham and Women's Hospital in Boston, Massachusetts, where he was an investigator in federally sponsored outcomes research as well as industry-sponsored clinical trials.

Levy is a graduate of the Yale School of Medicine, where he was chief medical resident and trained in internal medicine and nephrology. He completed fellowship training in clinical research through the Robert Wood Johnson Clinical Scholars Program at Yale.

JANET WOODCOCK, M.D., was named Acting Commissioner of Food and Drugs on January 20, 2021.

As Acting Commissioner, Dr. Woodcock oversees the full breadth of the FDA portfolio and execution of the Federal Food, Drug, and Cosmetic Act and other applicable laws. This includes assuring the safety, effectiveness, and security of human and veterinary drugs, vaccines and other biological products for human use, and medical devices; the safety and security of our nation's food supply, cosmetics, dietary supplements, products that give off electronic radiation; and the regulation of tobacco products.

Dr. Woodcock began her FDA career in 1984, joining the agency's Center for Biologics Evaluation and Research (CBER) as Director of the Division of Biological Investigational New Drugs, as well as serving as CBER's Acting Deputy Director for a period of time. She later became Director of the Office of Therapeutics Research and Review in CBER, which included the approval of the first biotechnology-based treatments for multiple sclerosis and cystic fibrosis during her tenure.

In 1994, Dr. Woodcock was named Director of the FDA's Center for Drug Evaluation and Research (CDER), overseeing the center's work that is the world's gold standard for drug approval and safety. There she led many of the FDA's drug initiatives, including introducing the concept of risk management as a new approach to drug safety; modernizing drug manufacturing and regulation through the Pharmaceutical Quality for the 21st Century Initiative; advancing medical discoveries from the laboratory to consumers more efficiently under the Critical Path Initiative; and launching the Safety First and Safe Use initiatives designed to improve drug safety management within and outside the FDA, respectively.

In 2004, Dr. Woodcock became Deputy Commissioner and Chief Medical Officer in the Office of the Commissioner. Later she took on other executive leadership positions in the Commissioner's Office, including Deputy Commissioner for Operations and Chief Operating Officer.

In 2007, Dr. Woodcock returned as Director of CDER until she was asked to lend her expertise to "Operation Warp Speed" for developing therapeutics during the COVID-19 pandemic, such as evaluating the potential benefits of monoclonal antibody treatments for certain COVID-19 patients. From late 2020, she split her time advising "Operation Warp Speed" on advancing COVID-19 therapeutics while also serving as the Principal Medical Advisor to the Commissioner on key priorities on behalf of the Office of the Commissioner.

Dr. Woodcock holds a Bachelor of Science in chemistry from Bucknell University (Lewisburg, PA), and a Doctor of Medicine from the Feinberg School of Medicine at Northwestern University Medical School (Chicago). She also completed further training and a fellowship in rheumatology, as well as held teaching appointments at the Pennsylvania State University and the University of California in San Francisco. She is board certified in internal medicine.

Dr. Woodcock has been bestowed numerous honors over her distinguished public health career, most notably: a Lifetime Achievement Award in 2015 from the Institute for Safe Medication Practices; the Ellen V. Sigal Advocacy Leadership Award in 2016 from Friends of Cancer Research; the Florence Kelley Consumer Leadership Award in 2017 from the National Consumers League; and the 2019 Biotechnology Heritage Award from the Biotechnology Innovation Organization and Science History Institute.

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 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, dis- cuss, and act on 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. The activities of the Forum are determined by its members, focusing on the major themes outlined below.

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."

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

INFRASTRUCTURE AND WORKFORCE FOR DRUG DISCOVERY, DEVELOPMENT, AND TRANSLATION

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

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In 2011, the Forum on Drug Discovery, Development, and Translation established an initiative to address challenges facing the U.S. clinical trials enterprise and to engage stakeholders in an open discussion of potentially transformative strategies to improve the efficiency and effectiveness of clinical trials. This workshop, *Envisioning a Transformed Clinical Trials Enterprise in the United States: Establishing an Agenda for 2020*, was held on November 7-8, 2011. A summary of this workshop is provided below, followed by a recap of the work background and objectives.

Workshop summary. There is growing recognition that the United States' clinical trials enterprise (CTE) faces great challenges. There is a gap between what is desired – where medical care is provided solely based on high quality evidence – and the reality – where there is limited capacity to generate timely and practical evidence for drug development and to support medical treatment decisions. With the need for transforming the CTE in the U.S. becoming more pressing, the IOM Forum on Drug Discovery, Development, and Translation held a two-day workshop in November 2011, bringing together leaders in research and health care. The workshop focused on how to transform the CTE, and discussed a vision to make the enterprise more efficient, effective, and fully integrated into the health care system. Key issue areas addressed at the workshop included: the development of a robust clinical trials workforce, the alignment of cultural and financial incentives for clinical trials, and the creation of a sustainable infrastructure to support a transformed CTE. The full proceedings from this workshop can be accessed here: https://www.nap.edu/read/13345/chapter/1

Background. There is increasing recognition that the clinical trials enterprise in the United States faces substantial challenges impeding the efficient and effective conduct of clinical and translational research needed to support the development of breakthrough medicines. A gap exists between the desired state where medical care in the United States is provided solely based on high quality evidence and the reality of our limited ability to generate timely and practicable evidence. 85 percent of clinical decisions in the United States are not supported by high quality evidence. At the same time, U.S. clinical trials that generate medical evidence are becoming increasingly costly while experiencing greater setbacks. In addition, the shifting "footprint" of clinical trials toward sites outside of the United States prompts questions about the generalizability and applicability of the results of those clinical trials to the U.S. population and represents a competitive challenge for the United States.

The limited ability of the nation's clinical trials system to support drug development and evaluation exists within a broader context of a need for a "learning health system," where "knowledge generation is so embedded into the core of the practice of medicine that it is a natural outgrowth and product of the healthcare delivery process and leads to continual improvement in care." An essential component of such a learning health system is a robust and well-working clinical trials enterprise to support drug development; inform quality improvement; and support surveillance, international, and comparative effectiveness research.

Workshop Objectives:

- Frame the problem and discuss a vision for a clinical trials enterprise that is efficient and effective and fully integrated into the health delivery system of 2020. Define how the envisioned clinical trials enterprise differs from the current system and suggest approaches to transform our current system into a learning system.
- Consider the following core themes in framing an agenda to effect transformation of the U.S. clinical trials enterprise:
    - Providing a vision for a clinical trials enterprise in the health care system of 2020.
    - Developing a robust clinical trials workforce.
    - Aligning cultural and financial incentives.
    - Building an infrastructure to support a transformed clinical trials enterprise.