Forum on
DRUG DISCOVERY, DEVELOPMENT, and TRANSLATION

Envisioning a Transformed Clinical Trials Enterprise for 2030

A Four-Part Virtual Workshop

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

The National Academies of
SCIENCES • ENGINEERING • MEDICINE

Envisioning a Transformed Clinical Trials Enterprise for 2030

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Part 4: May 11, 2021

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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 (former)

Anita LaFrance Allen, University of Pennsylvania

Christopher P. Austin, Flagship Pioneering

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 (former)

Anantha Shekhar, University of Pittsburgh

Pamela Tenaerts, Medable

Christopher Yoo, Systems Oncology

The National Academies of SCIENCES ENGINEERING MEDICINE

Forum on DRUG DISCOVERY, DEVELOPMENT, and TRANSLATION

Envisioning a Transformed Clinical Trials Enterprise for 2030 – A Virtual Workshop

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

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 2011 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) provided an overview discussion on how an envisioned 2030 clinical trials enterprise may differ from the current system. It discussed key challenges and opportunities in improving person-centeredness and inclusivity, building resilience and transparency, and integrating new technologies.

- Part Two (February 9, 2021) considered achievable goals to enhance person-centeredness and inclusivity in the clinical trials enterprise; and discussed ways to improve public engagement and partnership.

- Part Three (March 24, 2021) considered approaches to build resilience, sustainability, and transparency. The discussion included 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 outlined in prior virtual workshop parts. Discussion in this part of the workshop will**

    - **Discuss practical short-term and long-term goals for ensuring thoughtful and deliberate partnership with and use of new technologies to improve the clinical trials enterprise; and**
    - **Consider specific action steps that stakeholders could individually take to support an envisioned change in the next 5 years (by 2025) and in the next 10 years (by 2030).**

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

Workshop Part 4: May 11, 2021

Practical Applications for Technology to Enhance the Clinical Trials Enterprise

11:00a.m. – 3:00p.m. 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 THE ROAD TO 2030: AN ATLAS FOR CHANGE

Moderator: JENNIFER GOLDSACK
Executive Director
Digital Medicine Society

11:10 a.m. **Keynote address**
AMY ABERNETHY
Former Principal Deputy Commissioner of Food and Drugs
U.S. Food and Drug Administration

11:25 a.m. **Frontline Experience: A Panel Discussion**
A perspective on patient burden and accessibility
TARA HASTINGS
Senior Associate Director of Patient Engagement
Michael J. Fox Foundation

A perspective on digital law
JAN BENEDIKT BRÖNNEKE
Director Law & Economics Health Technologies
health innovation hub

A perspective on improving software and experience for clinical trial sites
BRADFORD HIRSCH
Chief Executive Officer
SignalPath Research

11:55 a.m. **Charge to the Breakout Groups**

12:00 p.m. **"Lightning Round" Breakout Discussion Groups** *(25 min)*

The breakout groups will be assigned one of the two following goals and asked to discuss practical applications and partnerships with new technologies that can address key priority challenges, opportunities aligned with this goal that will move us towards the clinical trials enterprise envisioned for 2030. See associated breakout discussion guides for more detail.

- **GOAL 1**: **Enable a more person-centered and easily accessible clinical trials enterprise.** This also relates to the vision of the Clinical Trial Transformation Initiative for 2030: https://www.ctti-clinicaltrials.org/transforming-trials-2030
- **GOAL 2: Simplify trials (less data collection, fewer site visits) and lower costs while still generating high-quality data and robust answers to relevant research questions.**

12:25 p.m. **Breakout group wrap up**

FIRESIDE CHAT

12:30 p.m. **Fireside chat**
MARK MCCLELLAN
Director
Duke-Margolis Center for Health Policy

AMY ABERNETHY, *Moderator*
Former Principal Deputy Commissioner of Food and Drugs
U.S. Food and Drug Administration

1:00 p.m. **BREAK** *(30 min)*

SESSION II THE ROAD TO 2030: A CALL TO ACTION

Moderator: ANITA ALLEN
Henry R. Silverman Professor of Law, Professor of Philosophy
University of Pennsylvania Carey Law School

1:30 p.m. **Frontline Experience: A Road Already Travelled**
JANICE CHANG
Chief Operating Officer
TransCelerate BioPharma Inc.

PAM TENAERTS
Chief Scientific Officer
Medable, Inc.

1:50 p.m. **Charge to the Breakout Groups**

2:00 p.m. **"Lightning Round" Breakout Discussion Groups** *(25 min)*

The breakout groups will be assigned one of the two following goals and asked to discuss practical applications and partnerships with new technologies that can address key priority challenges, opportunities aligned with this goal that will move us towards the clinical trials enterprise envisioned for 2030.

- **GOAL 3: Establish a clinical trials enterprise that is diverse, equitable, and inclusive.**
- **GOAL 4: Establish a national network of community-based clinical trial sites.**

2:25 p.m. **Breakout group wrap up**

CLOSING PANEL

2:35 p.m. **A "North-Star" Vision of What Is Possible**
ANDY CORAVOS
Chief Executive Officer and Co-Founder
Elektra Labs

ERIC PERAKSLIS
Chief Science and Digital Officer
Duke Clinical Research Institute

SAM ROOSZ
Co-founder and CEO
Crescendo Health

2:55 p.m. **Workshop wrap-up**
STEVEN GALSON, *Workshop Co-chair*
Senior Vice President, Research & Development
Amgen, Inc.

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

3:00 p.m. **ADJOURN**

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Planning Committee Biographies

CO-CHAIRS

STEVEN K. GALSON (co-chair), M.D., M.P.H. senior vice president, Research and Development, joined Amgen in 2010 as vice president, Global Regulatory Affairs and in 2014 became senior vice president managing global regulatory and safety. In January 2021 he stepped down from this position to focus on leading the company's COVID response activities. Galson is also on the Executive Committee of the Clinical Trial Transformation Initiative and a Trustee of the Keck Graduate Institute. Prior to Amgen, Galson was senior vice president for Civilian Health Operations and chief health scientist at Science Applications International Corporation. 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. 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 internationally-known oncologist, health data expert and digital health leader. Her >500 publications span real-world data & evidence, clinical trials, patient reported outcomes, clinical informatics, health policy and patient-centered care. Most recently, Dr. Abernethy was Principal Deputy Commissioner and Acting Chief Information Officer of the US Food & Drug Administration, serving from February 2019 to April 2021. Dr. Abernethy initiated multiple critical efforts during her tenure including FDA's technology and data modernization action plans and FDA's efforts to leverage real-world data & evidence to address critical questions during the COVID-19 pandemic.

Dr. Abernethy was Chief Medical Officer and Chief Scientific Officer at Flatiron Health from July 2014 to January 2019. Before joining Flatiron, Dr. Abernethy was Professor of Medicine at Duke University School of Medicine, and directed the Center for Learning Health Care in the Duke Clinical Research Institute and Duke Cancer Care Research Program in the Duke Cancer Institute. For more than 20 years, she has pioneered the development of technology platforms to spur novel advancements in clinical care, including the development of systems by which linked clinical data can support tracking cancer care, drug development, personalized medicine and scientific discovery. She has served as an appointee to multiple National Academy of Medicine committees, as Chair of the Health Data Research UK International Advisory Board, on the Board of the Personalized Medicine Coalition, and President of the American Academy of Hospice & Palliative Medicine.

Dr. Abernethy went to the University of Pennsylvania as an undergraduate, and then medical school at Duke, where she also did her Internal Medicine residency, a year as Chief Resident, and her hematology/oncology fellowship. She has her PhD from Flinders University in Australia, focused on evidence-based 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 a CEO-Partner at Flagship Pioneering in Cambridge, MA. In that role, he serves as CEO of one of Flagship's franchise companies, and advises on the operation and creation of other Flagship entitites. Before joining Flagship in 2021, Dr. Austin served for almost a decade as the first permanent director of the National Center for Advancing Translational Sciences (NCATS) at the NIH, where he formulated the strategic vision and scientific directions of the new center, and led its efforts in developing, demonstrating, and disseminating scientific and operational advances in translational science to get more treatments to more patients more quickly. Before NCATS, Austin was at the National Human Genome Research Institute at NIH, where he founded a variety of translational programs focused on technologies to derive biological insights and therapeutic potential from the human genome sequence. Austin came to NIH in 2002 from Merck, where his work focused on genome-based discovery of novel targets and drugs, with a particular focus on common complex neuropsychiatric diseases. He did his clinical training in internal medicine and neurology at Massachusetts General Hospital, research fellowship in genetics at Harvard, M.D. from Harvard Medical School, and A.B. in biology from Princeton University.

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, Pharm.D., 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 is 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, where he served for two years. 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 led 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 the Chief Scientific Officer at Medable, where she will direct research to help identify, implement and make ubiquitous responsible decentralized trial strategies. Dr. Tenaerts brings more than 30 years of experience in clinical trials, as a researcher and academic, in medical device research operations, a hospital based site administrator, and physician, most recently serving as executive director of the Clinical Trials Transformation Initiative (CTTI), a multi-stakeholder public private partnership to improve quality and efficiency in clinical trials at Duke University. She sit on the board of the Society of Clinical trials, the Scientific Leadership Council of the Digital Medicine Society, participates on the Good Clinical Trial Collaborative, and is a member of the National Academies of Science and Medicine: Forum on drug discovery, development and translation. She received her MD from the Catholic University in Leuven and her MBA from the University of South Florida.

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 & Moderator Biographies

SPEAKERS

AMY ABERNETHY, M.D., Ph.D, is an internationally-known oncologist, health data expert and digital health leader. Her >500 publications span real-world data & evidence, clinical trials, patient reported outcomes, clinical informatics, health policy and patient-centered care. Most recently, Dr. Abernethy was Principal Deputy Commissioner and Acting Chief Information Officer of the US Food & Drug Administration, serving from February 2019 to April 2021. Dr. Abernethy initiated multiple critical efforts during her tenure including FDA's technology and data modernization action plans and FDA's efforts to leverage real-world data & evidence to address critical questions during the COVID-19 pandemic.

Dr. Abernethy was Chief Medical Officer and Chief Scientific Officer at Flatiron Health from July 2014 to January 2019. Before joining Flatiron, Dr. Abernethy was Professor of Medicine at Duke University School of Medicine, and directed the Center for Learning Health Care in the Duke Clinical Research Institute and Duke Cancer Care Research Program in the Duke Cancer Institute. For more than 20 years, she has pioneered the development of technology platforms to spur novel advancements in clinical care, including the development of systems by which linked clinical data can support tracking cancer care, drug development, personalized medicine and scientific discovery. She has served as an appointee to multiple National Academy of Medicine committees, as Chair of the Health Data Research UK International Advisory Board, on the Board of the Personalized Medicine Coalition, and President of the American Academy of Hospice & Palliative Medicine.

Dr. Abernethy went to the University of Pennsylvania as an undergraduate, and then medical school at Duke, where she also did her Internal Medicine residency, a year as Chief Resident, and her hematology/oncology fellowship. She has her PhD from Flinders University in Australia, focused on evidence-based medicine.

JAN BENEDIKT BRÖNNEKE, LL.M., is Director Law & Economics of Health Technologies of the German health innovation hub (hih) – the Federal Health Ministry's think tank on the digitalization of healthcare. As a trained lawyer and economist with a research background in medical law, health technology assessment and medical device regulation he is leading, among others, the hih's projects on matters of access, regulation, evaluation and reimbursement of digital technologies within the German statutory health insurance. In this position he was closely supporting the development and implementation of the German Digital Healthcare Act, the Patient Data Protection Act and other legislative activities regarding digitalization of the German health care system. Before joining hih, he worked for the Federal Joint Committee on quality assurance in hospitals and private practices as well as a manager for a Berlin based law firm specialized on the law of medical devices and pharmaceuticals.

With said background, Jan bridges the gap between disciplines as well as between regulatory authorities and private actors such as technology developers, doctors and hospitals.

JANICE CHANG is the Chief Operating Officer at TransCelerate BioPharma Inc. Janice has been involved with the organization since its inception. In her current position, Janice works closely with the CEO and the Board of Directors to shape the long-term strategic vision and priorities for the organization and its 30+ initiatives. Janice defines and guides TransCelerate's overall external engagement strategy with global health authorities, governmental agencies, industry groups, and TransCelerate's country network spanning across 30 countries. She has accountability overseeing TransCelerate's corporate operations and works closely with her team to drive strategic delivery of TransCelerate's portfolio.

Janice also actively participates in various cross-stakeholder global discussions to help evolve our R&D paradigm. Most recently she joined the Advisory Council for HL7 International's Vulcan Accelerator. Vulcan is a global strategic effort to bring together stakeholders across the translational and clinical research community to align on data exchange standards to bridge existing gaps between clinical care and clinical research, enabling more effective acquisition, exchange and use of healthcare data in translational and clinical research.

With a background of 20+ years of experience leading initiatives in large pharma and biotech companies, Janice has experience spanning across regulatory, clinical, and manufacturing. Janice is passionate in driving meaningful change across our ecosystem and not settling for the status quo. She believes in reimagining the way we advance innovative medicine and advocates for the power of open collaboration across stakeholder groups.

ANDY CORAVOS is the co-founder and CEO of Elektra Labs, building a digital medicine platform with a focus on digital biomarkers for decentralized clinical trials, and a Member of the Harvard-MIT Center for Regulatory Sciences. Formerly, Andy served as an Entrepreneur in Residence at the FDA working in the Digital Health Unit (DHU), focusing on the Pre-Cert program and policies around software and AI/ML. Previously, she worked as a software engineer at Akili Interactive Labs, a leading digital therapeutic company. Before grad school, Andy worked at KKR, a private equity firm, and at McKinsey & Company, a management consulting firm, where she focused on the healthcare industry. She serves on the Board of the Digital Medicine Society (DiMe), and she's an advisor to the Biohacking Village at DEF CON.

TARA HASTINGS is the Senior Associate Director of Patient Engagement at the Michael J. Fox Foundation. Tara works with both the Parkinson's disease community and key stakeholders across the research and drug therapeutic landscape to ensure the patient perspective is present. She guides MJFF's efforts to foster collaboration and provide guidance on how to meaningfully capture and include patient insights, experiences, desires and preferences at all phases of development, through mechanisms such as innovative technology platforms, pre-competitive consortia and education initiatives. Tara holds a BA from Columbia University and MA from the University of Virginia.

BRADFORD HIRSCH, M.D., M.B.A., Co-Founder and Chief Executive Officer of SignalPath Research, a company leveraging technology to make clinical trials more efficient, effective and available. He is also a medical oncologist and principal investigator. Prior to his present focus, he held leadership roles in technology companies and academics including Flatiron Health and the Duke University School of Medicine. Across all of his roles, he has focused on the use of data and novel technologies to advance the frontier in medicine.

He received his B.A. from the University of Pennsylvania, M.D. from UT Southwestern in his hometown of Dallas, Tx, and completed his fellowship and M.B.A. at Duke University. From a clinical perspective, he focuses on treatment and research of genitourinary cancers. He has over 50 publications in the peer reviewed literature, continues to speak regularly on topics of medicine and technology, and serves on the ASCO Cancer Research Committee, Journal of Cancer Clinical Informatics Editorial Board, National Quality Forum Cancer Committee, Parkland Hospital Foundation Board of Directors, and National Outdoor Leadership School Advisory Board.

MARK MCCLELLAN, M.D., PH.D., is the Robert J. Margolis Professor of Business, Medicine, and Policy, and founding Director of the Duke-Margolis Center for Health Policy at Duke University. With offices in Durham, NC and Washington, DC, the Center is a university-wide Duke initiative that is nationally and internationally-recognized for research, evaluation, implementation, and educational initiatives to improve health policy and health, most recently in its COVID-19 response. The Center integrates Duke's expertise in the social, clinical, and analytical sciences alongside engagement with health care leaders and stakeholders, to develop and apply policy solutions that improve health, health equity, and the value of health care locally, nationally, and worldwide.

Dr. McClellan is a doctor and an economist who has addressed a wide range of strategies and policy reforms to improve health care, including payment reform to promote better outcomes and lower costs, methods for development and use of real-world evidence, and strategies for more effective biomedical innovation.

At the center of the nation's efforts to combat the pandemic, Dr. McClellan is the co-author of a roadmap that details the steps needed for a comprehensive COVID-19 response and safe reopening of our country. His current work on

responding to the COVID-19 public health emergency spans virus containment and testing strategies, reforming health care toward more resilient models of delivering care, and accelerating the development of therapeutics and vaccines.

Before coming to Duke, he served as a Senior Fellow in Economic Studies at the Brookings Institution, where he was Director of the Health Care Innovation and Value Initiatives and led the Richard Merkin Initiative on Payment Reform and Clinical Leadership. He also has a highly distinguished record in public service and academic research.

Dr. McClellan is a former administrator of the Centers for Medicare & Medicaid Services (CMS) and former commissioner of the U.S. Food and Drug Administration (FDA), where he developed and implemented major reforms in health policy. These include the Medicare prescription drug benefit, Medicare and Medicaid payment reforms, the FDA's Critical Path Initiative, and public-private initiatives to develop better information on the quality and cost of care. He has also previously served as a member of the President's Council of Economic Advisers and senior director for health care policy at the White House, and as Deputy Assistant Secretary for Economic Policy at the Department of the Treasury.

Dr. McClellan is the founding chair and a Senior Advisor of the Reagan-Udall Foundation for the FDA, serves on the ICER Advisory Board, and is a member of the National Academy of Medicine (NAM). He chairs the NAM's Leadership Council for Value and Science-Driven Health Care, co-chairs the Guiding Committee of the Health Care Payment Learning and Action Network, and is a research associate at the National Bureau of Economic Research. He is also a Senior Advisor on the faculty of the University of Texas Dell Medical School and is an independent director on the boards of Johnson & Johnson, Cigna, Alignment Healthcare, and PrognomIQ. He was previously an associate professor of economics and medicine with tenure at Stanford University, and has twice received the Kenneth Arrow Award for Outstanding Research in Health Economics.

ANAEZE CHIDIEBELE OFFODILE II, M.D., M.P.H., is the Executive Director for Clinical Transformation and an Assistant Professor in the Department of Plastic Surgery at MD Anderson Cancer Center. Collaborating with key clinical and operational leaders throughout the institution, he is helping define, align and implement a high-level roadmap for clinical and economic transformation in support of MD Anderson's vision to deliver high-value cancer care. He is also a non-resident scholar in domestic health policy at the Baker Institute, a non-partisan think tank on the campus of Rice University. His scholarship is focused on examining the subjective and material impact of patient-borne treatment-related costs ("financial toxicity") and the interaction between vertical integration and the delivery of high-value care. He has received several national awards for his research work as well as competitive funding from the Doris Duke Charitable Foundation, University Cancer Foundation, and Blue Cross Blue Shield Affordability Cures Consortium. He is currently the 2019-2021 Gilbert Omenn Fellow at the National Academy of Medicine (NAM).

A graduate of Columbia University College of Physicians and Surgeons, he completed surgical training at Brigham & Women's Hospital (General Surgery), Lahey Clinic (Plastic Surgery) and MD Anderson Cancer Center (Microvascular fellowship). Dr. Offodile also received an MPH in health policy from the Bloomberg School of Public Health at Johns Hopkins University. Lastly, he previously served as a senior advisor to the Director of the Patient Care Models Group (Christina Ritter) at the Center for Medicare and Medicaid Innovation.

ERIC PERAKSLIS, PH.D., is the Chief Science and Digital Officer at the Duke Clinical Research Institute. He was previously a Rubenstein Fellow at Duke University, where his work focused on collaborative efforts in data science that spanned medicine, policy, engineering, computer science, information technology, and security. Immediately prior to his arrival at Duke, Dr. Perakslis served as Chief Scientific Advisor at Datavant, Lecturer in the Department of Biomedical Informatics at Harvard Medical School, and Strategic Innovation Advisor to Médecins Sans Frontières.

Dr. Perakslis was Senior Vice President and Head of the Takeda R&D Data Science Institute, where he built an integrated institute of more than 165 multi-disciplinary data scientists serving all aspects of biopharmaceutical R&D and digital health. Prior to Takeda, Eric was the Executive Director of the Center for Biomedical Informatics and the Countway Library of Medicine, an Instructor in Pediatrics at Harvard Medical School, and a faculty member of the Children's Hospital Informatics Program at Boston Children' Hospital.

During his time at HMS, Dr. Perakslis focused on the approval of the Department of Biomedical Informatics as a full academic department, the development of the NIH Undiagnosed Diseases Network, industry collaborations, leading the

technology efforts for multiple Ebola response programs, and building active research programs in medical product development, regulatory science, and cybersecurity.

Prior to HMS, Dr. Perakslis served as Chief Information Officer and Chief Scientist (Informatics) at the U.S. Food and Drug Administration. In this role, he authored the first IT Strategic Plan for FDA and was responsible for modernizing and enhancing the IT capabilities as well as in silico scientific capabilities at FDA.

Prior to his time at FDA, Dr. Perakslis was Senior Vice President of R&D Information Technology at Johnson & Johnson Pharmaceuticals R&D and member of the Corporate Office of Science and Technology. While at J&J, he created and open-sourced the tranSMART clinical data system, which is now being freely used by hundreds of healthcare organizations. During his 13 years at J&J, he also held the posts of VP R&D Informatics, VP and Chief Information Officer, Director of Research Information Technology, and Director of Drug Discovery Research. Prior to working at J&J, Dr. Perakslis was the Group leader of Scientific Computing at ArQule Inc.

Dr. Perakslis has served on the editorial board of Cancer Today magazine and as the Associate Editor for Novel Communications for the Journal of Therapeutic Innovation and Regulatory Science. He has also served on Science and Technology Advisory Committees and in leadership roles for the American Society of Clinical Oncology, NuMedii, Precision for Medicine, the Survivor Advisory Board at the Cancer Institute of New Jersey, the Kidney Cancer Association, OneMind4Research, and the Scientist - Survivor program of the American Association for Cancer Research. Internationally, he has served as the Chief Information Officer of the King Hussein Institute for Biotechnology and Cancer in Amman, Jordan.

Dr. Perakslis has a PhD in chemical and biochemical engineering from Drexel University. He also holds BSChE and MS degrees in chemical engineering.

SAM ROOSZ, M.B.A., is Chief Executive Officer and co-founder of Crescendo Health, a healthcare technology company that supports trial sponsors in integrating RWE into their study designs. Sam previously co-founded Datavant, the leading vendor of privacy-preserving record linkage, where he served as General Manager of Life Sciences. Sam also previously held roles at Natera, Element Science, and Putnam Associates. He holds a degree in Molecular and Cellular Biology from Harvard University and an MBA from Stanford.

JONATHAN WATANABE, PHARMD., PHD., BCGP, is Professor of Clinical Pharmacy and Associate Dean of Assessment and Quality at the University of California Irvine School of Pharmacy & Pharmaceutical Sciences and National Academy of Medicine Emerging Leader in Health and Medicine Scholar. He was a contributor to the National Academy of Sciences, Engineering, and Medicine (NASEM) Making Medicines Affordable: A National Imperative consensus report and is a current member of the NASEM ad hoc committee on Implications of Discarded Weight-Based Drugs. Dr. Watanabe employs real-world data to develop policy solutions to improve patient care, augment population health, and reduce medical costs. Watanabe focuses on improving access to evidence-driven medication use and pharmacist-directed patient care. He serves as an advisor to the California Health Benefits Review Program for the California State Legislature. His research on safe and effective medication use has been cited in enacted legislation efforts. He served as an investigator, faculty, and fellowship director for the federal Health Resources and Services Administration funded San Diego Geriatrics Workforce Enhancement Program and is a current investigator for the California Tobacco-related Disease Research Program. Dr. Watanabe was the inaugural recipient of the University of Washington/Allergan Global Health Economics and Outcomes Research Fellowship. Professor Watanabe served as a clinical consultant at the San Diego Program of All-inclusive Care for the Elderly (PACE) Clinic. He is an advisor to the Joint Commission on pain management and assessment standards in long-term care. He received his BS from the University of Washington (UW). He received a doctor of pharmacy (PharmD) from the University of Southern California. He received an MS and PhD from the UW Comparative Health Outcomes, Policy, and Economics (CHOICE) Institute. He is a Board-Certified Geriatric Pharmacist (BCGP).

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, discuss, 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 revolution- ary developments of new scientific techniques, therapeu- tic 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.

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.

Forum on Drug Discovery, Development, and Translation

Robert Califf (Co-Chair)
Verily Life Sciences and Google Health

Gregory Simon (Co-Chair)
Kaiser Permanente Washington Health Research Institute

Christopher Austin
Flagship Pioneering

Linda Brady
National Institute of Mental Health, NIH

Barry Coller
The Rockefeller University

Thomas Curran
Children's Mercy, Kansas City

Richard Davey
National Institute of Allergy and Infectious Diseases, NIH

Katherine Dawson
Biogen

James Doroshow
National Cancer Institute, NIH

Jeffrey Drazen
New England Journal of Medicine

Steven Galson
Amgen Inc.

Carlos Garner
Eli Lilly and Company

Julie Gerberding
Merck & Co., Inc.

Deborah Hung
Harvard Medical School

Esther Krofah
FasterCures, Milken Institute

Lisa LaVange
University of North Carolina Gillings School of Global Public Health

Leanne Madre
Clinical Trials Transformation Initiative, Duke University

Ross McKinney, Jr.
Association of American Medical Colleges

Joseph Menetski
Foundation for the NIH

Arti Rai
Duke University School of Law

Klaus Romero
Critical Path Institute

Kelly Rose
Burroughs Wellcome Fund

Susan Schaeffer
The Patients' Academy for Research Advocacy

Joseph Scheeren
Retired

Anantha Shekhar
University of Pittsburgh School of Medicine

Jay Siegel
Retired

Ellen Sigal
Friends of Cancer Research

Lana Skirboll
Sanofi

Amir Tamiz
National Institute of Neurological Disorders and Stroke, NIH

Ann Taylor
AstraZeneca

Pamela Tenaerts
Medable, Inc.

Joanne Waldstreicher
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For more information, please visit:

NATIONALACADEMIES.ORG/DRUGFORUM

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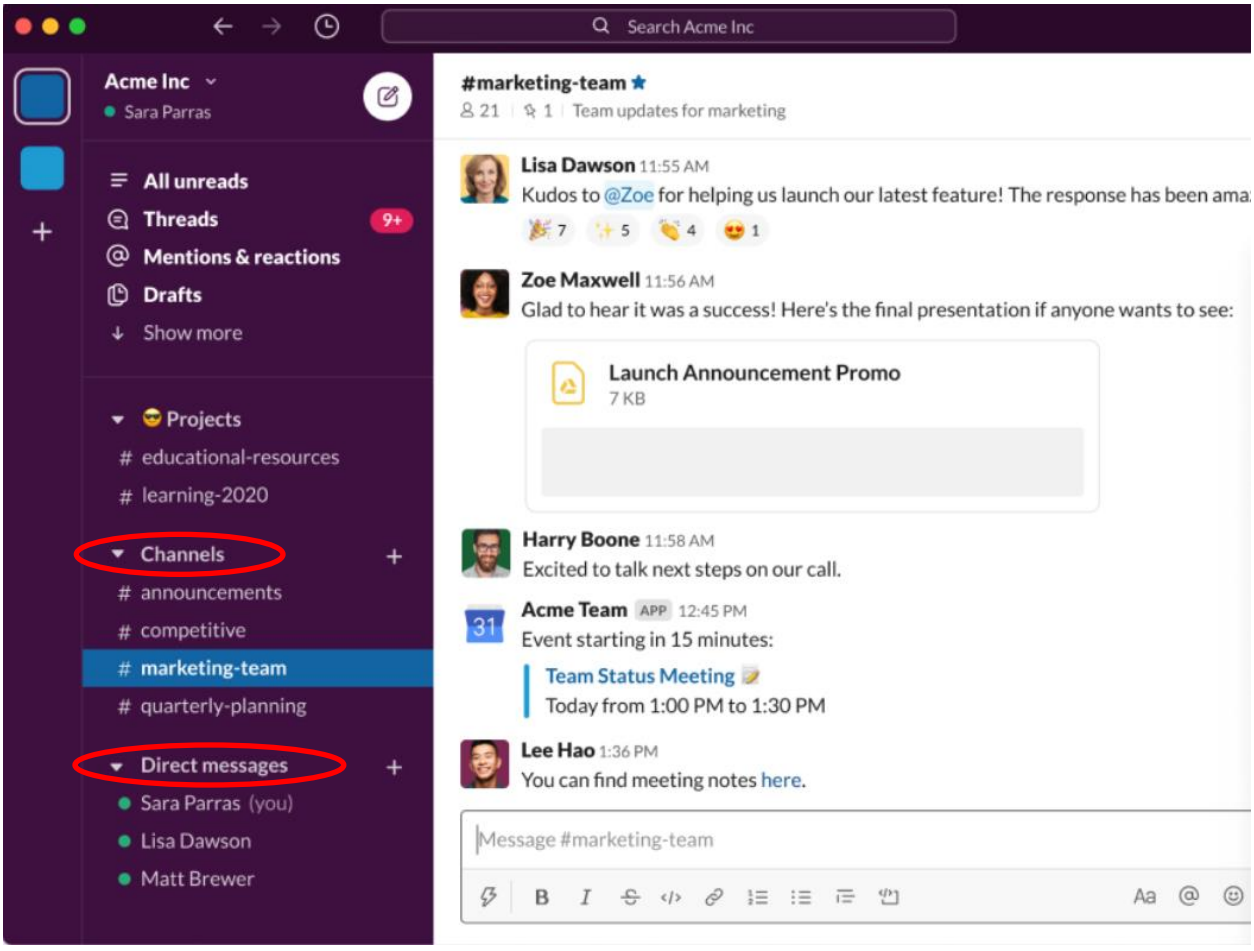
What is Slack?

Slack is an online discussion forum. You can join Slack to ask questions and exchange ideas before, during, and after the workshop meetings. We have asked workshop moderators, speakers, breakout discussion participants, and the interested members of the public to join so that you are able to ask/answer questions and engage with each other in ongoing conversations.

To understand how Slack really works, it helps to know how all the pieces fit together.

Here's a snapshot of what a typical Slack workspace looks like:



The sections on the left that will be most pertinent are **Channels** and **Direct Messages**. The white area to the right is where you will see the individual messages and conversations between members in the channel that you are viewing.

Channels

Your Slack workspace is comprised of **channels**. We have created a channel for each meeting in this four-part workshop. There is also a #water-cooler channel for everyone to introduce themselves and meet each other.

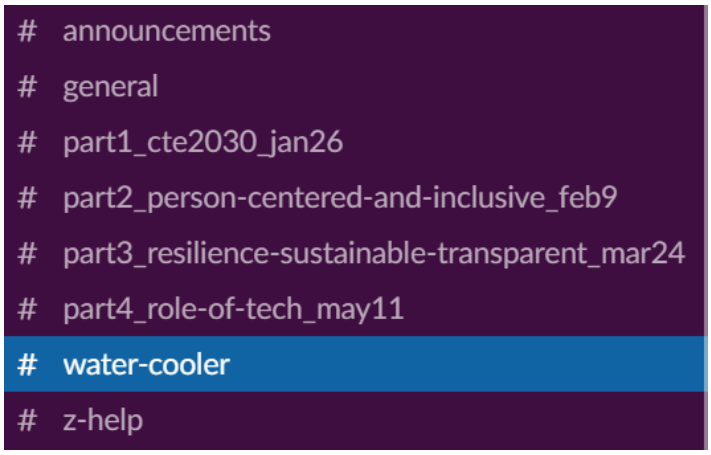
In these channels, you can add links to materials, send messages to everyone in this group during the meeting, and keep the conversation going between or after meetings.

There are also two "housekeeping" channels:

- The #announcements channel is where staff will post updates or news.
- The #z-help channel is where you can reach out to staff for technical help or ask questions related to the workshop.

NASEM staff will still communicate critical items via email, so you will not miss anything major if you choose not to join, but others have found Slack to foster engaging and lively conversations both during and following workshop meetings. We encourage you to please try it out.

The ClinicalTrials_2030 Slack workspace is set up with these channels:



Direct Messages

You can also reach out to others privately using **direct messages**. We encourage you to use the channels as much as possible, but if you have a small question or note that concerns only one or two others, you may use this feature to discuss with them. (If you have a logistical question, you can either post in the #z-help channel, or reach out directly by typing @Julie Liao.)

Quick Guide to Using Slack

Joining the ClinicalTrials_2030 Slack Workspace

This tool can only be effective if members of the group are willing to participate. We recommend that you log into Slack on your browser on a regular basis, or download the desktop application to receive notifications.

Notifications are completely customizable, so you can set the frequency and type of notifications you would like to receive.

Account Invitation: You can join the ClinicalTrials_2030 workspace with this invite link: https://join.slack.com/t/clinicaltrials2030/shared_invite/zt-n960kcpv-jeNbanIYzcRJ~JjbIdmZ0w

Access: By joining the workspace, you will immediate gain access to all the channels listed above.

Notifications: You will be able to customize when and how you are notified and for what. You can choose to be notified of activity within specific groups, when your name is mentioned, or for key words. If you choose to download the desktop application, you may opt to receive desktop notifications. If you use the web-application, notifications are only visible when you sign in. There is also a mobile application with customizable push-notifications.

Slack Dos and Don'ts

DO

- post news and information that are related to the workshop and discussions
- post links to relevant material
- ask questions or continue the discussions from the workshop
- approach staff for assistance or guidance
- treat all committee members and staff on this channel with respect

DON'T

- post solicitations for private work (events, job openings, etc.)
- create private channels without including at least 1 staff member

For a more detailed how-to guide with pictures, please visit here.

Envisioning a Transformed Clinical Trials Enterprise for 2030

A Four-Part Virtual Workshop

Health Affairs Blog Series: Envisioning A Transformed Clinical Trials Enterprise For 2030

1. Transforming Clinical Trials: A New Vision For 2030 *by Marilyn Metcalf and Rob Weker*
2. Driving Towards More Inclusive Clinical Trials By 2030: Action Without Strategy is Aimless, Strategy Without Action is Powerless *by Silas Buchanan*
3. A Future Of Trusted Clinical Trials: Communication Strategies To Encourage Trust And Transparency *by Brian Southwell*
4. The Future of Clinical Trials: How Will New Technologies Affect The Lives Of Participants? *by Eric D. Perakslis, Andrea Coravos, and Sam Roosz*

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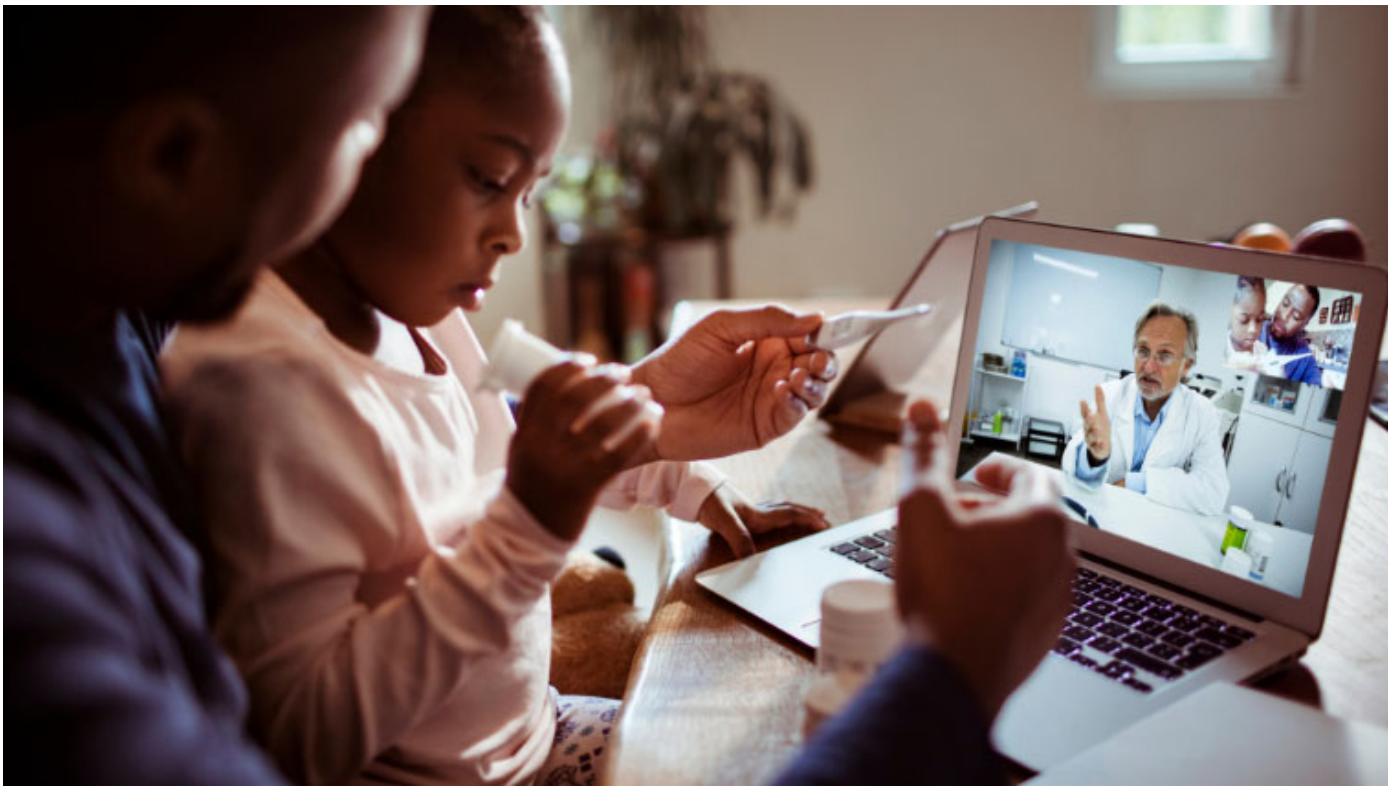
Transforming Clinical Trials: A New Vision For 2030

Marilyn Metcalf, Rob Weker

MAY 5, 2021 **DOI:** 10.1377/hblog20210503.897529



Editor's Note

This post inaugurates the Health Affairs *Blog short series, "Envisioning A Transformed Clinical*

Trials Enterprise For 2030." The series explores ideas for advancing a clinical trials enterprise that is more efficient, effective, person-centered, inclusive, and integrated into the health delivery system of 2030 than is currently the case. The series is being published in conjunction with a four-part public workshop, also titled Envisioning a Transformed Clinical Trials Enterprise in 2030 and *convened under the auspices of the National Academies Forum on Drug Discovery, Development, and Translation. Posts in this series are published by* Health Affairs *Blog with the support of the National Academies, but they represent the opinions of the authors and do not necessarily represent the views of the Forum on Drug Discovery, Development, and Translation, the National Academies, or any other organization; these posts do not constitute reports or products of the National Academies.*

The year is 2030. My wearable device detects a measurement that could signal a health problem. It immediately notifies me and my primary care physician. All possible associated health problems are searched, assessed according to my history, and ranked probabilistically using artificial intelligence. A timely diagnosis determines my medical condition.

My primary care physician and I explore possible treatment options and together decide that a clinical trial may be best suited for me. A top medical specialist and health system are identified to meet my health requirements and preferences (e.g., type of treatment, location of study site). I obtain a second opinion from another specialist regarding my diagnosis and clinical options.

My medical team, caregiver, and I agree on a treatment plan. I understand my course of action. A nurse practitioner coordinates and integrates my medical care. Any medications I take outside of my study visits are automatically supplied directly to me (as appropriate). When I take the trial medicine it is automatically tracked with smart technology, e.g., automated uploading of weight from a digital scale or data from an insulin pen.

The study site takes a multidisciplinary approach to integrating my medical treatment considering, e.g., my other health conditions, nutrition, and psychological well-being. My entire medical team meets to discuss my case and develops a tailored, integrated treatment plan. My caregiver is also supported and integrated as a key player in my treatment.

Automated mechanisms are in place to capture my patient-reported data with detail and insight while mitigating the effort to report them. Regulators will use these patient-reported metrics as a key part of their deliberation and approval process. Researchers may also use this trove of data to identify important questions not addressed by existing trials.

A patient navigator—a health professional who works with me to coordinate my care—helps me manage financial resources; facilitates communication between myself, my family, and medical providers; and addresses my medical questions and psychosocial needs. My caregiver and I are connected to an experienced patient who has walked the journey for my

disease. I receive frequent updates; the 'burden' on me and my caregiver is minimized as much as possible. At the end of treatment, my post-treatment plan is well documented, and understood by me, my caregiver, and my medical team. Follow up monitoring and ongoing communication is appropriate and based on my personal preferences.

Making This Future Attainable For All Patients

Timely integration of clinical trial information along with medical information and psychosocial considerations does not occur seamlessly. The data are dispersed and often the information is communicated to the patient by many experts. But the vision described above is achievable by better integrating systems and through capabilities already within reach. For patients who are well insured, have access to major health care facilities, and see health care providers who are aware of or participate in clinical trials, this is a plausible vision.

But how do we ensure this vision is inclusive of *all* patients? We must consider the patients who have limited access to health information, who are not well insured, who do not have health care providers or whose physicians do not have familiarity with or access to clinical trials or cutting-edge therapies. It is important that information, choices, and care are available for all patients, understandable to them, and accessible with or without internet connections and smart phones. Health care research and the access it offers to newer therapies needs to move further into local communities, partnering with trusted centers of communication. This cannot be a one-way broadcast of information. It needs to be a true conversation among patients, caregivers, community leaders, health care providers, and researchers. There are some models in place (e.g., the Center for Sustainable Health Care Quality and Equity—promoting sustainable healthy communities in every zip code), but they are not yet standard practice.

Advancing The Science Of Patient Input

Patients today face many challenges when it comes to finding the right diagnoses, treatments, and/or clinical trials. Patient input has aided tremendously in understanding and overcoming these challenges. For example, Fox Insight, an online research study for Parkinson's diseases supported by the Michael J. Fox Foundation for Parkinson's Research, enables study participants to contribute information about their family and medical history, current medical conditions, quality of life, and activities of daily living. This information helps researchers to build a natural history of each patient's disease.

Better aligning priorities for patient involvement in medical product research and development with regulatory decision-making regarding early disease detection, disease management, and treatment would create a more direct translation of research to practice. Further, converting traditionally anecdotal patient input into rigorous, credible evidence for

use by a broad range of stakeholders could improve patients' experiences and outcomes in clinical research and care.

A 2018 workshop hosted by the Forum on Drug Discovery, Development, and Translation at the National Academies of Science, Engineering, and Medicine (the Forum) examined knowledge gaps and other barriers that hinder advancement of the science of patient input into medical product research and development. The list of barriers was further reviewed and prioritized by participants in the Advancing the Science of Patient Input Action Collaborative; this was an ad hoc activity associated with the Forum to identify and prioritize specific areas of research that—if effectively addressed—could lead to more evidence-based and effective patient input.

To promote scientific rigor, patients need to be involved from early research planning throughout the development of medicines, in clinical aspects and also nonclinical aspects such as the delivery of medicines through appropriate devices. More person-centered research is emerging through patients' advice on protocols, informed consent forms, educational materials, and study logistics. This makes clinical trials more feasible and accessible for patients through improved information sharing that is meaningful and understandable (e.g., through more comprehensible informed consent forms); it lowers the hurdles patients must overcome (e.g., through reduced travel burdens to study sites via fewer procedures arranged to better fit patients' schedules). Accessibility can be further enhanced by ongoing engagement with patients and sharing of insights among stakeholders throughout the drug research and development lifecycle.

A Path Forward

Ultimately the patient wants timely, transparent, understandable information to make decisions about participating in a study. To build a more relevant clinical trials enterprise, outside-the-box thinking needs to incorporate meaningful patient input throughout the research lifecycle.

For Patients Who Participated In Clinical Research, Provide Opportunities For Engagement, Ongoing Connection, And Long-Term Follow-Up

Involving patients long-term provides two advantages. First, regardless of that patient's outcome, they are not left feeling that the research community is "done with me." As a patient explained in an internal team meeting on clinical trial design, "I still have my life. Keep me informed. Don't just drop me. Keep a psychological connection." Second, a growing pool of knowledgeable contributors can be asked, under appropriate agreements, for their ideas and input into improving clinical research for both patients and providers.

Create And Support More Opportunities For Involvement In Clinical Research

For The Network Of Researchers And Health Care Providers

Include more material about clinical research in the education of health care providers. Provide opportunities for patients to teach and learn. Create opportunities for practicing health care providers to participate in clinical research, e.g., as sub-investigators, through observational or pragmatic studies, or in other ways that allow broader reach into diverse and underserved communities. “Diversity” here can refer to race and ethnicity, economic factors, or rural versus urban; the idea is to address any combination of systemic inequalities fueled by lack of funding, infrastructure, and other resources that continues to widen gaps between patients’ health care access and outcomes.

Finally, support these opportunities by assessing and equitably implementing solutions that decrease the burden on health care providers, who often must balance research commitments against their existing clinical care. Use lessons from the recent COVID-19 pandemic to “decentralize” clinical trials. Allow local sites to collect study samples from patients and send them to a central screening or laboratory site for analysis, reducing variability in study test results while increasing participation of health care providers and patients in more distributed facilities. Ship clinical trial treatments to local sites or directly to patients and include their local health care providers in monitoring clinical trial patients’ health.

For Patients And Health Care Providers, Create An Open Dialogue To Address Questions And Concerns About The Differences Between Research And Clinical Care

Clinical research may involve randomization of patients by treatment groups to test new approaches that may or may not be better than the standard of care. Clinical care aims to provide each patient with the best available treatment for that individual. Establish a sense of trust and open dialogue with patients and providers to address their underlying questions and concerns. Involve them as partners in developing research agendas. Ensure that patients and providers have a clear understanding of what happens during a trial, the type of health care patients will receive, any costs related to enrolling in a trial, the benefits and risks associated with participation, and any potential long-term cost implications of trial participation and treatment, including coverage by payers. Finally, as part of maintaining an ongoing connection, share access to the collected data with the patient and inform them of findings from the trial.

The challenges before us are not new; we have discussed them for decades. Now it is surely time to act, to learn from the extraordinary circumstances and opportunities created by the COVID-19 pandemic, including the lessons of our connections as humans around the globe. In the following post in this series, Silas Buchanan (link live on Thursday, May 6) addresses

the challenges of reaching out to underserved communities such as Black Americans who have particular reason to distrust the clinical trial enterprise.

Creating a person-centered health care system necessitates a more equitable health care system. Our human community should now require better cooperation among stakeholders, more concerted focus on health priorities at a global level, and more flexibility in meeting local health needs.

Authors' Note

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Driving Towards More Inclusive Clinical Trials By 2030: Action Without Strategy is Aimless, Strategy Without Action is Powerless

Silas Buchanan

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Editor's Note

This post is part of the Health Affairs *Blog short series, "Envisioning A Transformed Clinical Trials Enterprise For 2030." The series explores ideas for advancing a clinical trials enterprise that is more efficient, effective, person-centered, inclusive, and integrated into the health delivery system of 2030 than is currently the case. The series is being published in conjunction with a four-part public workshop, also titled Envisioning a Transformed Clinical Trials Enterprise in 2030 and convened under the auspices of the National Academies Forum on Drug Discovery, Development, and Translation. Posts in this series are published by* Health Affairs *Blog with the support of the National Academies, but they represent the opinions of the authors and do not necessarily represent the views of the Forum on Drug Discovery, Development, and Translation, the National Academies, or any other organization; these posts do not constitute reports or products of the National Academies.*

Systemic racism is the monster under our collective bed, built into our most fundamental social structures and institutions. For members of underserved communities and particularly community members of color, it impacts where we live, the quality of our education, how likely we are to encounter unethical policing, our access to voting—and, crucially, our ability to access quality health care.

Consequently, as we plan towards 2030, any authentic discussion about improving inclusive engagement in the clinical trials enterprise must, at a minimum, acknowledge systemic racism as a precursor. For instance, coronavirus disease 2019 (COVID-19) negatively affects all of us in obvious ways. However, the pandemic *disproportionately* affects many of us in

ways that are not always easy to see, measure, understand, or even acknowledge.

Systemic racism is a root cause of health disparities and therefore one of the social determinants of health (SDOH). SDOH are the conditions in the places where people are born, live, learn, work, pray, play, age and die. These conditions explain, at least in part, why some community members are healthier than others and why many underserved community members are disproportionately impacted by a wide variety of chronic illnesses.

Morbidity and mortality rates for community members of color are exponentially higher across almost every chronic illness. These chronic illnesses, in turn, leave underserved community members more susceptible to infectious diseases. In fact, the Centers for Disease Control and Prevention (CDC) reports that community members of color have contracted and died from COVID-19 in disproportionate numbers, and continue to do so.

These disappointing and complicated cycles diminish trust in our health care system generally; they further damage the already fragile trust between the clinical trials enterprise and underserved communities specifically. Even well-intended actions toward improving inclusiveness in clinical trials that are not embedded in culturally appropriate strategies will not restore this trust. As we contemplate what a more positive and inclusive clinical trials enterprise in 2030 might look like, breaking these cycles while equitably addressing the SDOH could be our most important task.

The Challenges Of Persuading Leaders Of Underserved Communities To Trust That The Clinical Trial Enterprise Has Changed

I have had hundreds of conversations about addressing SDOH and eliminating health disparities with community leaders and members of underserved communities across the country. I have worked extensively at the intersection of underserved community outreach and engagement, and I have created mutually beneficial and equitable partnerships between underserved communities and public and private health care stakeholders. My organization worked in partnership with the AME Church to build the official health information dissemination and data collection platform for its 2,000 congregations and 2 million members worldwide. This platform combines a community-facing web portal for congregants and community members with a private, password-protected, socially networked backend to connect and support the work of the clergy and health ministry staff.

In my private conversations with the leadership of both secular and nonsecular community-based organizations, the discussions invariably turn to SDOH—specifically, systemic racism and lack of trust. We spend a lot of time talking through the tactics and strategies needed to improve the health and wellbeing of their communities, including how to best create the

partnerships mentioned above. In our conversations, the health care system and the clinical trials enterprise are generally seen as inextricably connected; when discussing the clinical trials enterprise as a separate entity, the levels of distrust are even higher. The wounds and emotions around the history of inequitable, racist, and cruel treatment of communities of color within the clinical trial enterprise are still raw.

While some faith and community-based leaders readily acknowledge the importance of their community members participating in clinical trials, significant frustrations remain. Questions that come up during these conversations include: "Why should the onus be on us to just suddenly become more trusting? What has changed within the clinical trials enterprise to earn our trust? Show me the list of what precisely is different from before." These frustrations do not seem completely intractable, but they are steeped in a well-earned and historically based lack of trust, exacerbated by the inability of stakeholders throughout the clinical trials enterprise to consistently and clearly articulate why they should engender trust. And, logically, a lack of trust by the leadership of influential faith and community-based organizations can easily translate to community members as well.

The Importance Of Equitable Partnerships

Based on my work, a more inclusive clinical trials enterprise in 2030 will largely be defined by the number of equitable partnerships created with underserved faith and community-based organizations. The path to those potential partnerships begins by building trust with organization leaders. Fortunately for the clinical trials enterprise, many of these leaders are recognized as valuable subject matter experts on community engagement and they are generally known, liked, and trusted by their community members. Their organizations remain historically embedded in their communities and have dedicated missions to improve the health and wellbeing of community members. Many of them would be qualified and ideal choices to direct, consult and/or collaborate in the co-creation of powerfully effective, culturally appropriate, community outreach and engagement strategies and campaigns.

Creating equitable and ongoing connections would in turn create significant opportunities for co-learning. The clinical trials enterprise would have opportunities to ease frustrations and consistently answer questions. There would be ample opportunities to show community leaders and their community members exactly what has changed about the clinical trial process. There would be the chance not only to ask, "How can we do the research better?" but also, "*What* research should we be doing?" There would also be opportunities to create an active workforce pipeline to identify, recruit, educate, and encourage the growth and development of Principal Investigators of color.

Conversely, and not to be understated, there would be ongoing opportunities for community leaders to educate clinical trialists, researchers, trial sponsors, and other key stakeholders on the histories of marginalization and distrust felt by communities of color. Organizational

leaders could facilitate transparent discussions about where the real and perceived power lies in their own communities. They could lead and deepen the understanding of key stakeholders throughout the clinical trials enterprise on how best to build on existing community strengths by respecting and leveraging local knowledge and resources. These community leaders are seeking genuine partnerships with health care organizations, including those within the clinical trials enterprise, that protect the wellbeing, interests, and rights of communities and community members of color in longer-term, measurable, and mutually beneficial ways.

Stakeholders Too Often See Community Leaders As Commodities, Not Partners

However, today many community-based organizations find themselves inundated with opportunities for "sponsorship" from health care payer or provider, government, academic and pharmaceutical stakeholders—and, every election cycle, from politicians. Yet, community-based organizations are generally only seen as commodities, not equitable partners, by the health care stakeholders seeking to engage them. As a result, they are leveraged for their market access, market intelligence, and ability to collect data, but receive little benefit in return. Many times, community-based organizations are not factored into the sponsor's budget and instead are asked to volunteer staff and personal time to distribute information. Moreover, they receive negligible usable data back about their very own communities. This hampers the ability of community-based organizations to seek local foundation funding to strengthen their own work and organizations. And once again, valuable community-based organizations find themselves underappreciated, underutilized, and underserved.

Now is the time to resolve to address this imbalance and clearly recognize the attainable, mutual benefit of working equitably on culturally appropriate strategies and actions that are both purposeful and powerful. While the discussion here has focused on communities of color, the need for equitable partnerships may be extended to include other underserved populations, including those living in rural or impoverished urban areas, persons in poverty or low socioeconomic status, the uninsured, vulnerable children and families, and individuals with chronic health conditions.

Looking Ahead

As we look towards 2030, I optimistically envision a much more inclusive and equitable health care and clinical trials enterprise:

One that supports the development of a robust, culturally and linguistically appropriate, web-based social network that has trusted faith- and community-based organizations at its core.

One that strengthens and empowers the messengers by connecting community-based organizations with each other and to the large numbers of underserved community members

they represent.

One that does not view underserved community-based organizations simply as sponsorable commodities, but as valuable partners with critical insights and qualified leadership.

One that consistently acknowledges the history of race-based inequities from slavery until today and is committed to change.

One that is committed to educating, training, and developing Principal Investigators of color.

One that seeks equitable partnerships to drive culturally and linguistically appropriate campaigns that improve the health and wellbeing of underserved community members.

One that unites the resolve to take action with strategies to drive towards a more inclusive clinical trials enterprise in ways that will always be easy to see, easy to measure, easy to understand, and even easy to acknowledge.

Author's Note

Silas Buchanan is the principal at the Institute for eHealth Equity and chief executive officer of OurHealthyCommunity, a social impact firm with the mission to improve the health, economic opportunity, education, and civic engagement of underserved communities through the innovative use of technology. The author thanks the staff of the National Academies Forum on Drug Discovery, Development, and Translation, including C. Shore, A. Wagner Gee, and J. Liao for contributing to the development of this blog piece. Additional thanks go to Alexandra McGregor, PhD, GlaxoSmithKline.

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A Future Of Trusted Clinical Trials: Communication Strategies To Encourage Trust And Transparency

Brian Southwell

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Editor's Note

This post is part of the Health Affairs *Blog short series, "Envisioning A Transformed Clinical Trials Enterprise For 2030." The series explores ideas for advancing a clinical trials enterprise that is more efficient, effective, person-centered, inclusive, and integrated into the health delivery system of 2030 than is currently the case. The series is being published in conjunction with a four-part public workshop, also titled Envisioning a Transformed Clinical Trials Enterprise in 2030 and convened under the auspices of the National Academies Forum on Drug Discovery, Development, and Translation. Posts in this series are published by* Health Affairs *Blog with the support of the National Academies, but they represent the opinions of the authors and do not necessarily represent the views of the Forum on Drug Discovery, Development, and Translation, the National Academies, or any other organization; these posts do not constitute reports or products of the National Academies.*

The year is 2030. The clinical trials enterprise continues to produce important scientific advancement and does so by enrolling people who represent those most directly affected by diseases. Trial staff advertise and recruit to their studies through robust partnerships with media outlets and community-based organizations. We hear few reports about participant hesitancy relative to past decades.

Prospective trial participants have credible information resources available in their local communities to address concerns. The results of trials are widely accessible to general audiences along with useful information about the context and interpretation of those results. Media commentators celebrate the institutions that run clinical trials as important partners in improving community wellbeing.

The COVID-19 pandemic has highlighted the importance of timely drug discovery and development for public health, and the challenges of clinical trials as social endeavors. Trials are social: they involve recruitment of people as participants; they also involve communication with patients, their caregivers, and their families about results and study implications. People may talk with one another about misgivings and amplify concerns. An individual's decision process for participating in clinical trials or accepting the trial results is based on a variety of cognitive and emotional factors. Overlooking the human component of trials' practice and translation risks falling short on health, wellbeing, and equity.

How might we embrace a person-centered perspective regarding communication activities in support of clinical trials, so that we can achieve the vision for 2030 outlined above? Fundamentally, those of us working in institutions that sponsor, implement, or

report on trials must consider key concepts—including the notions of trust and transparency—more explicitly and carefully than has been the case historically.

Trust

What does it mean for trial participants and public audience members to trust the institutions that design and run clinical trials, or to trust those who report on trial results? Trial professionals and those working with them sometimes think about trust in terms of intellectual credibility, but there are additional dimensions of trust, including reliability (or consistency) and perceptions of shared interest.

The iterative nature of science can lead to an evolving understanding of what we know about a given phenomenon. At the same time, a stream of seemingly inconsistent headlines about scientific findings can undermine public trust in the clinical trial enterprise. Rushing too quickly to announce a particular trial result and later issuing a revision based on updated information, or not explaining the nuances in the trial results, can result in outcomes perceived as contradictory and therefore unreliable by general publics. Providing clear, lay-friendly explanations regarding the possibility of study iteration and adjustments in trial results as early as possible can pave the way for public acceptance.

Perceptions of shared interest are also tied to perceptions of trust. To what extent do patients see their fate and future concerns as bound up with those who are organizing and implementing trials? In the context of a pandemic, many may see global benefits of an effective vaccine, although even in that situation patients might be worried that profits are being prioritized over public health. In other contexts, instances of shared interest might be even less clear to patients and could require explicit communication efforts, to listen and to articulate how organizations and patients share interest in study outcomes. Taking that step might mean explicitly articulating goals that patients and trial organizers share. In the case of communicable disease, for example, trial organizers might state their own interests in reducing the need for quarantines and social isolation in the future and resuming various forms of economic activity, interests that are shared by many potential participants.

Building trust between organizations and the public holds tremendous promise for public cooperation and support. One example of this has been the work of the COVID-19 Prevention Network which has involved various community members in the process of trial protocol development as a way of ensuring substantive community engagement. Moreover, trusted relationships between organizations and patients can serve as an

antidote to the current proliferation of misinformation by reducing patient tendency to wander the Internet in search of information rather than turning to trusted sources.

Those who sponsor and implement trials also can signal shared interest by working with organizations that have been overlooked. There might be capacity for improving trials within existing community organizations even if those organizations have not been historically involved in studies of specific diseases. Consider the need to encourage participation in clinical trials by Black patients. Rather than creating altogether new structures and initiatives, it may be more effective to invest in partnerships with long-standing institutions that are already serving various communities, such as the National Medical Association or medical programs at Historically Black Colleges or Universities.

Transparency

What about transparency? Publicizing details of trials can be a step in the right direction. Offering privacy assurance for participants in trials that involve digital data collection technology is another important step. But the physical availability of information is not the same as public understanding of that information, as colleagues and I argued recently. Optimizing transparency requires accounting for variability in the audience's scientific background and literacy. To what extent do people understand key concepts such as clinical endpoints, for example?

Available literature would suggest we have a long way to go in ensuring that people have the background knowledge—or what we might think of as foundational mental models—needed to understand the latest announcements. Organizations working on clinical trials could collectively encourage the development and distribution of educational resources to explain the science of trials, laying a foundation of scientific literacy and facilitating future communication.

If transparency involves communication between trials staff and their audiences, listening also should be part of our vision. We can develop new workforce roles to better incorporate listening to communities as a formal component of the trials enterprise. Such a move would build on existing partnerships with communities, such as Michele Andrasik of the University of Washington has engaged in for years in working with community groups to encourage HIV vaccine trial participation. If public health and health care organizations are geographically situated in various communities, for example, these organizations can band together and form a collaborative effort in terms of listening.

If we need to assess and respond to emergent community concerns about trials research, investment in the listening capacity of public health agencies and health care organizations could yield tremendous future benefits. Monitoring social media, attending community meetings, establishing partnerships with local leadership, and collecting and responding to commonly asked questions can offer a crucial foundation for public engagement; yet that work will not happen without effort and personnel assignments. In practice, this perspective calls for greater attention to the capacity for social science research and communication skills in hiring staff to work on the clinical trials enterprise.

A Vision For Trust And Transparency As Key To Future Trials Success

The resources needed in the 21st century will involve more than test tubes and data analysis programs. Building trust, encouraging functional transparency, and ensuring the resiliency and sustainability of future study efforts require investment in relationships and acknowledgement of long-standing community organizations. The work necessary to optimize future trials recruitment and public translation of results should be happening now, before the next public health crisis takes place. We can build social ties, assess community needs, and refine our ability to explain abstract concepts through a variety of communication efforts not conventionally associated with clinical trials science. Doing that could greatly benefit the future of clinical trials research and translation.

Author's Note

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The Future of Clinical Trials: How Will New Technologies Affect The Lives Of Participants?

Eric D. Perakslis, Andrea Coravos, Sam Roosz

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Editor's Note

This post is part of the Health Affairs *Blog short series, "Envisioning A Transformed Clinical Trials Enterprise For 2030." The series explores ideas for advancing a clinical trials enterprise that is more efficient, effective, person-centered, inclusive, and integrated into the health delivery system of 2030 than is currently the case. The series is being published in conjunction with a four-part public workshop, Envisioning a Transformed Clinical Trials Enterprise in 2030, convened under the auspices of the National Academies Forum on Drug Discovery, Development, and Translation. Posts in this series are published by* Health Affairs *Blog with the support of the National Academies, but they represent the opinions of the authors and do not necessarily represent the views of the Forum on Drug Discovery, Development, and Translation, the National Academies, or any other organization; these posts do not constitute reports or products of the National Academies.*

There has been no shortage recently of opinion pieces penned on the future of clinical trials that speak of advancing sensor and digital technologies, of new approaches in methodology and data science, of patient engagement that bridges the gaping chasm between the incentives that drive fee-for-service medicine and the brutal exposure of the patient journey.

We will not do that here. Instead, we would like to introduce you to: Mary, David, Sarah, and Tim. We invite you to follow their lives from the present to a future world where technological advances have been fully embraced by all stakeholders, enabled by supportive policies, and integrated into the health care system. We hope you will get to know them and consider joining them on their journeys.

Mary and David

*It's **2020.** Mary is a young, college-educated Latina mother living in southern Texas. Her husband, David, is her high school sweetheart and the sole provider for Mary and their twin toddler sons. David has steadily gained weight over the last few years and resembles his dad more every day. David's father recently passed away from liver cancer. Mary has been worried ever since because so many in her community have died of this cancer.*

David has a great job but has had to work day and night since COVID-19 struck. This has been good for them financially but the double shifts, the junk-food meals, and the lack of sleep all worry Mary. She wonders if liver cancer runs in families, and whether there's any way to know if David is more likely to have this disease – and if so, just how much risk does he have? She asked the family doctor who mentioned high rates of liver cancer in the Latinx community, but had no explanations or recommendations beyond general "healthy living". She brings the issue up with her sister, a nurse, but her sister cautions her about risks related to participating in medical research, including loss of privacy, exploitation, and possible side effects of experimental treatments.

Latinos are twice as likely to die of liver cancer as are non-Hispanic white people, and the role

that genetic factors play in this difference has not been studied fully. The vastly disproportionate enrollment of white men in clinical trials has led to potentially harmful gaps in knowledge about health experiences in diverse communities. Studies show that outreach, education, and native-language videos can be effective in increasing awareness and inclusive participation of diverse communities in biobanking and clinical trials. However, recruiting study participants from among patients in the primary care setting may be a better approach as it would build upon the trusted clinician-patient relationship to empower patients to form opinions on the risks of participation (e.g., via modernized informed consent processes). Incentives to encourage and enable primary care practices to participate in clinical trial enrollment, coupled with adaptation of intuitive and cloud-based technologies that are designed with user input to facilitate the process, can realize this vision. In the future, community primary care must be an active participant in clinical trial recruitment.

Now it's 2030. *The triumphant "clang" of a bell fills the waiting room, the most beautiful sound Mary has ever heard. David is cancer-free. Six months earlier a normal checkup had led to a hurried CT scan after David's blood had shown potential tumor DNA with markers specific to the liver. David's primary care provider, whom David and his wife have known for more than a decade, was able to provide David and his family some reassurance with background material on potential treatments (including clinical trials) and a referral to a trusted community oncologist colleague.*

When David first visited his community oncologist, the doctor quickly assembled his medical history through the use of David's Universal Patient Identifier. The software then suggested some potential treatment options: some traditional approaches based on the National Comprehensive Cancer Network (NCCN) guidelines but also clinical trials for which David met the eligibility criteria. After a discussion with the doctor, David expressed interest in targeted therapy that addressed a mutation common in the Latinx population. David reviewed an interactive consent that combined audio and visuals to clearly convey the benefits, risks, and expectations of the study, as well as how his data would be used (he chose English but was glad to see a Spanish option). He felt comfortable with his clinical protocol, and was able to share his next steps with his wife and friends confidently.

After the tumor had shrunk in size, he went into surgery to remove its last remnants and energetically rang the bell after recovery. Proud of his successful battle, he enrolled in a pragmatic observational study that would track his long-term outcomes via the data generated through his normal care.

Sarah

It's ***2020.*** *Sarah, a young mother of three and a BRCA2 "previvor," lives in rural Maine. Happily married and financially stable, Sarah gets good health care but lives with fear and stigma about the cancer that runs in her family. She participates in online communities and enjoys the*

support she receives there, but also feels geographically isolated from all the great cancer research she hears about that's taking place in Boston, Texas, and New York City.

She also worries about the trustworthiness of some of the things she reads on her BRCA2 Facebook Group and is not really sure what to believe sometimes. She would love to participate in a clinical trial that may provide answers and help her cope and is open to anything from prevention trials to familial genetic studies—really, anything that seems legitimate. Her primary care physician did not know of any such studies taking place nearby.

The primary barriers to patients participating in clinical trials are logistical, practical, geographical, and financial. In the future, once clinical trial recruitment is ubiquitous within primary care medicine, the logistical barriers must come down. Remote enrollment and trial support based on robust digital health tools can broaden and ensure equitable access to biomedical research for all by providing:

- decentralized trial capabilities that enable online recruitment and telehealth for clinical encounters
- flexible and adaptable informed consent and patient compacts
- executable study protocols
- advanced privacy protection

Once clinical trials are no longer tied to a limited set of "hub" hospitals, a broader pool of patients can be reached and recruited, reducing barriers to establishing and maintaining a broader set of registries.

*It's **2030**. Sarah feels the familiar buzz in her jacket pocket and she sneaks a glance at the email she just received. The study had successfully closed and its results were ready for her to review! For the past five years, Sarah had participated in an observational trial for those with BRCA2 mutations. The trial was run out of a large hospital in Boston, though she never actually had to make a trip. She'd dutifully filled out surveys on her phone, wore the trial smartwatch that was mailed to her to collect health-related data, and had even donated blood to the nurse who had visited her home in Maine over the last several years (thankfully she was largely healthy in that time).*

Now that the results were finally being published, the trial had reached out to share a preprint of the research paper, a layperson summary, and a private link to all of Sarah's records and data from the study. She smiled and thought of her sister and two daughters as she opened the study summary, eager to learn.

Tim

*It's **2020**. Tim is a young CPA living in Nevada. He's the youngest partner at his new firm,*

recently married, and looking forward to starting a family once his wife finishes law school. A childhood diagnosis of autosomal recessive polycystic kidney disease (ARPKD, a rare disease affecting about 1 in 20,000 births) led to Tim receiving a kidney transplant at age 10 that allowed him to enjoy healthy kidney function. However, he had recently started experiencing unusual fatigue and abdominal pain, and, after visiting his doctor, learned that his ARPKD was now placing him on the road to liver failure.

After a few days of scary introspection, Tim vows to his wife and to himself that he will fight this disease and win, both for himself as well as for the family he wants to start. He wonders how he could participate in research but is told there are no active studies for ARPKD and the disease has been largely neglected by the pharmaceutical industry. Even if a trial becomes available, Tim is concerned he will be given the placebo, which will do nothing for his disease. Tim is searching for any hope of a treatment that will help him and the children he's looking forward to raising.

In the future, clinical trials will be conducted on rare diseases for which running successful studies had long been viewed as logistically or economically infeasible. Sensor-based data collection and better real-world data tools will enable researchers to collect more data from a smaller pool of patients, and synthetic control arms will further reduce enrollment needs and ensure patients can access the investigational therapies. New protocols and wearable devices could enable the use of high-frequency in-home monitoring data, allowing a study to recruit faster and require fewer participants, which is critical for rare disease communities.

*It's **2030.** Tim grunts as he catches the baseball his daughter threw to him. How could a 5-year-old throw so hard? Maybe he should have worn a glove. On returning to the house, he grabs his autoinjector and is once again grateful that a small biotech had made a bet on Tim and others with his condition. He was one of just a couple dozen patients in the study that kicked off in 2025—and he ultimately never needed to worry about getting the placebo as the clinical protocol had instead used historical data from patients like him as a point of comparison.*

He and the other trial enrollees were spread out around the country, and only had to travel to Rochester once a year. The rest was done from his home—from the delivery of the drug to the wristwatch he wore that was somehow able to use light to track his bilirubin and creatinine levels. He and others in the trial fought ARPKD and overcame it so triumphantly that no one else ever needed to worry about managing this disease again.

We hope you enjoyed meeting Mary, David, Sarah, and Tim. Their futures are possible, but not inevitable. These technological capabilities already exist, but more needs to be done to integrate and broaden their use. They are counting on you to make that happen.

Authors' Note

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