NATIONAL ACADEMIES Sciences Engineering Medicine

The Food and Drug Administration's Accelerated Approval Process for New Pharmaceuticals

A Virtual Workshop

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Speaker Biographies

Halima Amjad,* M.D., Ph.D., M.P.H., is an Assistant Professor of Medicine in the Division of Geriatric Medicine and Gerontology at Johns Hopkins University School of Medicine. She has a clinical and research focus on dementia, providing clinical care at the Johns Hopkins Memory and Alzheimer's Treatment Center. Her research examines early diagnosis and support for older adults with dementia, funded by the National Institute on Aging. Arising from experience with her own father's early onset dementia, she serves on the Board of Directors of the Association for Frontotemporal Degeneration. She also serves on the Maryland Virginia I. Jones Alzheimer's Disease and Related Disorders Council and the Editorial Board of the Journal of the American Geriatrics Society. After medical school at Drexel University College of Medicine, Dr. Amjad completed her residency in internal medicine at Yale-New Haven Hospital and a clinical and research fellowship in geriatric medicine at Johns Hopkins. She also holds a Ph.D. in Clinical Investigation from the Johns Hopkins University Bloomberg School of Public Health.

Ginny Beakes-Read is Executive Director, Global Regulatory and R&D Policy at Amgen. She leads the GRR&D policy group, which works to shape the regulatory environment in ways that support innovative drug development and patient access to new therapies. The team works with Regulatory, Development, Commercial, Health Policy, Government Affairs, and other departments on a variety of regulatory and policy matters. Beakes-Read joined Amgen from Eisai, where she led the Global Regulatory Policy team for 8 years. For her last 2 years at Eisai, Beakes-Read was the Executive Director/Special Counsel, Regulatory Strategy and Law, where she was a member of the Global Regulatory Affairs and Legal Departments.

Beakes-Read previously worked at Genentech, Inc., as Director, Regulatory Policy and Strategy, in the Washington, D.C. Office. Prior to her work at Genentech, she was at FDA where she was the Director, Division of Regulatory Policy II, Office of Regulatory Policy in CDER for 8 years. In that position she was responsible for the development of regulations affecting CDER, and was involved with crafting policy positions in areas such as follow-on biologics. Before her tenure at FDA, Beakes-Read was a U.S. Army JAG, working as a prosecutor and appellate attorney. Beakes-Read is also an RN and started her career as an intensive care nurse in the US Air Force. She holds B.S.N. and J.D. degrees from the University of Virginia.

Barbara E. Bierer,** M.D., a hematologist-oncologist, is Professor of Medicine at Harvard Medical School (HMS) and the Brigham and Women's Hospital (BWH). Dr. Bierer is the Faculty Director of the Multi-Regional Clinical Trials Center of BWH and Harvard (MRCT Center), a collaborative effort to improve standards for the planning and conduct of international clinical trials. She is also the Director of

the Regulatory Foundations, Ethics, and Law program at the Harvard Catalyst, and Director of Regulatory Policy for SMART IRB. She serves as Faculty in the Center for Bioethics, HMS, and Affiliate Faculty in the Petrie-Flom Center for Health Law at Harvard Law School. She is a co-founder of COVID-19 Collaboration Platform and of the non-profit Vivli, a global clinical research data sharing platform. From 2003 – 2014, Bierer served as Senior Vice-President, Research, BWH where she founded the Brigham Research Institute and the Brigham Innovation Hub. She is a past chair of SACHRP and has served or serves on the Board of Directors of AAHRPP, PRIMR, MSH, Vivli, North Star IRB, and the Edward P. Evans Foundation. She has authored over 275 publications. Bierer received her B.S. from Yale University and her M.D. from Harvard Medical School.

Gideon Blumenthal is a hematologist oncologist who is currently Vice President, Oncology Global Regulatory Affairs and Clinical Safety, Merck. He has overseen regulatory strategy for pembrolizumab across Genitourinary, Gastrointestinal, Head and Neck Cancer, and Hematologic Malignancies. In addition, he has overseen strategy for belzutifan in VHL and Renal Cell Carcinoma, and investigational agents including vibostolimab, favezelimab and nemtabrutinib. Prior to joining Merck in 2020, Dr. Blumenthal spent over a decade at the US Food and Drug Administration Oncology office, taking on increasing leadership responsibilities. He initially served as a medical reviewer, then clinical team leader, followed by Acting Deputy Director in the Office of Hematology Oncology Products and Associate Director for Precision Oncology, and most recently as Deputy Center Director of the Oncology Center for Excellence. Blumenthal trained in internal medicine at the University of Maryland School of Medicine, followed by a hematology oncology fellowship at the National Cancer Institute. He was an attending physician in the National Cancer Institute thoracic oncology clinic. He received numerous awards, including the 2018 American Society for Clinical Oncology Public Service Award. He has co-authored over 100 articles in the Oncology and Drug Development peer reviewed literature and has authored numerous book chapters.

Marc Buyse holds degrees from Brussels University (Belgium), Cranfield University (UK) and a ScD in biostatistics from the Harvard School of Public Health (USA). Prior to founding the International Drug Development Institute (IDDI) in 1991, he had worked at the European Organization for Research and Treatment of Cancer (EORTC) in Brussels and at the Dana Farber Cancer Institute in Boston. He is also the founder of CluePoints, a company dedicated to statistical monitoring of clinical trials, and an Associate Professor of biostatistics at Hasselt University in Belgium. His recent work focuses primarily on developing statistical methods for personalized medicine.

Jacqueline Corrigan-Curay, J.D., M.D., is the Principal Deputy Center Director of the Center for Drug Evaluation and Research (CDER) at the U.S. Food and Drug Administration. Most recently, she served as the Acting Center Deputy Director for Operations, directing center and agency-level priority and initiative programs and leading GDUFA III reauthorization negotiations.

Previously, Corrigan-Curay was director of CDER's Office of Medical Policy (OMP). In that role, she led the development, coordination, and implementation of medical policy programs and strategic initiatives. She worked collaboratively with other CDER program areas, FDA centers, and stakeholders on enhancing policies to improve drug development and regulatory review processes.

Corrigan-Curay brings to the position a unique legal, scientific policy, and clinical background with expertise in risk and scientific assessment, and clinical trial design and oversight. Before joining FDA, she served as supervisory medical officer with the Immediate Office of the Director, National Heart, Lung and Blood Institute (NHLBI) at the National Institutes of Health (NIH). She also served in director and acting director roles with the Office of Biotechnology Activities (OBA), Office of Science Policy at NIH, where she was executive secretary of the NIH Recombinant DNA Advisory Committee. She has held positions as an attending physician with the VA Medical Center, a policy analyst with the Congressional Office of Technology Assessment, and as a practicing attorney in Washington, D.C.

Corrigan-Curay earned her law degree from Harvard Law School, her medical degree from University of Maryland School of Medicine, and a bachelor's degree in history of science from Harvard/Radcliffe College in Cambridge, MA. She completed her training in internal medicine at Georgetown University Medical Center, where she also served as a clinical assistant professor of medicine. She has continued to practice internal medicine part-time at the Veterans Affairs Medical Center in Washington, D.C.

Donna R. Cryer, J.D., is Founder and Chief Executive Officer of Global Liver Institute (GLI), the premier patient-driven liver health nonprofit operating with offices and partnerships across five continents. Moved by her own experience as a 28-year liver transplant recipient, Cryer serves as a fierce advocate for the transformative potential of patient engagement in health policy, research, data, and system design.

Cryer's expertise and effectiveness in advancing the voice of patients in defining and designing equitable healthcare has been recognized by the United States Congress and the White House. In 2021, Cryer received both the Global Genes RARE Champions of Hope Founder's Award and the American Association for the Study of the Liver (AASLD) Distinguished Advocacy Service Award.

Cryer serves on the Boards of Directors for the Council of Medical Specialty Societies (CMSS), Sibley Memorial Hospital/Johns Hopkins Medicine, and the Color of Crohns and Chronic Illness (COCCI). She was the first patient to serve on the American Board of Internal Medicine Gastroenterology Specialty Board.

Cryer received an undergraduate degree from Harvard and a Juris Doctorate from the Georgetown University Law Center.

David L. DeMets, Ph.D. is currently the Max Halperin Professor of Biostatistics, Emeritus, and former Chair of the Department of Biostatistics and Medical Informatics at the University of Wisconsin â€“ Madison. He received his PhD in biostatistics in 1970 from the University of Minnesota. Following a postdoctoral appointment in the Division of Computer Research and Technology at the National Institutes of Health (1970-72), he spent ten years (1972-1982) at the National Heart, Lung and Blood Institute at the National Institutes of Health where he was a member of and later became chief of the Biostatistics Branch. 1982, he joined the University of Wisconsin and later founded the Department of Biostatistics and Medical Informatics which he chaired until 2009. In 2017, He became emeritus professor.

DeMets has co-authored four texts, *Fundamentals of Clinical Trials*, *Data Monitoring in Clinical Trials: A Case Studies Approach*, *Data Monitoring Committees in Clinical Trials: A Practical Perspective*, and *Statistical Methods for Clinical Trials*. He has served on numerous NIH and industry-sponsored Data Safety and Monitoring Committees for clinical trials in diverse disciplines. He served on the Board of Directors of the Society for Clinical Trials (1983-1987) American Statistical Association (1987-89), as well as having been President of the Society for Clinical Trials (1989) and President of the Eastern North American Region (ENAR) of the Biometric Society (1993). In addition, he was Elected Fellow of the International Statistics Institute in 1984, the American Statistical Association in 1986, the Association for the Advancement of Science in 1998, the Society for Clinical Trials in 2006, and the American Medical Informatics Association in 2008. In 2013, he was elected as a member of the Institute of Medicine, which is now the National Academy of Medicine.

DeMets' research interests include the design, data monitoring and analysis of clinical trials, especially large Phase III randomized clinical trials. He is well known for his work on sequential statistical methods for monitoring interim data for early evidence of intervention benefit or possible harm.

Scott S. Emerson, M.D., Ph.D., is Professor Emeritus of Biostatistics at the University of Washington. After receiving an undergraduate degree in physics and an M.D., both from the University of Virginia, Emerson earned a Master's degree in computer science (University of Virginia) and a Ph.D. in Biostatistics (University of Washington). He held faculty positions in the Department of Statistics at the University of Florida and in the Department of Statistics and Arizona Cancer Center at the University of Arizona prior to joining the faculty of the Department of Biostatistics at the University of Washington, serving as Graduate Program Director for six years. Emerson is active in research into methods for the design, conduct, and analysis of clinical trials. A major focus of his research has been in the use of sequential methods, both frequentist and Bayesian, in the monitoring and reporting of clinical trial results. Recent areas of research include the sequential monitoring of longitudinal studies, especially when the primary outcome is based on time to event, adaptive clinical trial designs, noninferiority trials, missing data in RCT, and nonparametric regression methods, especially as applied to survival data and ROC curve analysis. Computer programs that he developed for his research into group sequential methodology now form the backbone of S+SeqTrial, an S-PLUS module for group sequential trial design that has been ported to R as RCTdesign. He served as a member of the National Academies of Science Oversight Committee for Missing Data in Clinical Trials. His collaborations in clinical research include a broad spectrum of disease areas, including cardiovascular disease, oncology, diabetes, obesity, emergency medicine, liver disease, renal disease, pulmonary disease, infectious diseases, allergy, and rheumatology. He serves on a number of government and industry sponsored Data Safety Monitoring Boards (DSMBs) and clinical trial steering committees, as well as serving on FDA Advisory Committees. He is a Fellow of the American Statistical Association.

Holly Fernandez Lynch, J.D., M.B.E., is Assistant Professor of Medical Ethics and Law at the University of Pennsylvania. She pursues conceptual and empirical scholarship regarding clinical research ethics and regulation, access to investigational medicines outside clinical trials, and FDA pharmaceutical policy, especially early approval standards for diseases with unmet treatment needs. She is currently working on a qualitative study of barriers, facilitators, and alternatives to confirming benefit after FDA drug approval, funded by Arnold Ventures.

Fernandez Lynch is a board member of Public Responsibility in Medicine & Research (PRIM&R) and the American Society for Law, Medicine, and Ethics, as well as a fellow of the Hastings Center and a National Academy of Medicine Emerging Leader in Health and Medicine. She previously worked as an attorney in private practice, bioethicist serving NIH’s Division of AIDS, analyst with President Obama's Commission for the Study of Bioethical Issues, and executive director of the Petrie-Flom Center, Harvard Law School's bioethics and health law research program.

Lee A. Fleisher, M.D., was named the Chief Medical Officer and Director of the Center for Clinical Standards and Quality for the Centers for Medicare and Medicaid Services in July 2020. In this capacity, he is responsible for executing all national clinical, quality, and safety standards for healthcare facilities and providers, as well as establishing coverage determinations for items and services that improve health outcomes for Medicare beneficiaries. He is also Professor of Anesthesiology and Critical Care and Professor of Medicine at the University of Pennsylvania Perelman School of Medicine. From 2004 through July 2020, he was the Robert D. Dripps Professor and Chair of Anesthesiology and Critical Care and Professor of Medicine at the University of Pennsylvania. He remains a Professor of Anesthesiology and Critical Care and Medicine at the Perelman School of Medicine at the University of Pennsylvania and Senior Fellow of the Leonard Davis Institute.

Fleisher received his medical degree from the State University of New York at Stony Brook, from which he received the Distinguished Alumni Award in 2011. His research focuses on perioperative cardiovascular risk assessment and reduction, measurement of quality of care, decision making, implementation of cultural change and health policy. He has received numerous federal, industry and foundation grants related to these subjects and has published 175+ original articles, over 200 editorials,

reviews and book chapters, and nine books and collaborates with anthropologists, sociologists, as well as faculty from law, business and nursing.

Fleisher was Treasurer of the Board of Directors and Chair of the Finance Committee of the National Quality Forum. He was a member of the Care Transformation Forum (CTF) of the Health Care Payment Learning and Action Network (LAN) He is currently an Affiliated Faculty of the Quattrone Center for the Fair Administration of Justice at the University of Pennsylvania Carey Law School and a Senior Fellow of the Leonard Davis Institute of Health Economics. In 2007, he was elected to membership of the National Academy of Medicine (NAM) (formerly Institute of Medicine) and served on committees of the NAM.

Yasuhiro Fujiwara has been Chief Executive of Pharmaceuticals and Medical Devices Agency (PMDA) since April 1, 2019. He has been a Vice-chair of the International Coalition of Medicines Regulatory Authorities (ICMRA) since 2019. Dr. Fujiwara was previously Director-General, Strategic Planning Bureau of the National Cancer Center, and the Deputy Director, National Cancer Center Hospital. He is a medical oncologist, specializing in breast cancer. Between Jan 2011 to Feb 2013, he was a Deputy Secretary General of Office of Medical Innovation, Cabinet Secretariat of Japan, and led health policy issues regarding life science.

Rogério Gaspar,* Ph.D., is the Director of RPQ (Department of Regulation and Prequalification) at the World Health Organization (WHO). He was for the last 36 years an academic, with extensive international regulatory experience during the last 26 years. Previously, among other positions, he was Vice-President of INFARMED and member of the management board of the European Medicines Agency (2000-2002), Vice-Rector of the University of Lisbon (2013-2017), and member of the Executive Committee and Vice-President of the European Federation of Pharmaceutical Sciences (EUFEPS, 2009-2013 and 2016-2020). A researcher in nanomedicine since 1986, Gaspar has also been involved in regulatory science and health systems projects in recent years.

William K. Hubbard* served for many years at the U.S. Food and Drug Administration (FDA) before retiring in 2005, having joined the Department of Health and Human Services (HHS) in 1973. During his last 14 years of federal service, Hubbard was an FDA Associate Commissioner responsible for, among other things, FDA's regulations and policy development. In his time at FDA, he spearheaded many drug-related initiatives, including the creation of the Prescription Drug User Fee Act and the redesign of the label for over the counter (OTC) drugs. Hubbard was instrumental in the development of the FDA's Accelerated Approval Program, for which he received the HHS Distinguished Service Award, HHS's highest award, from the HHS Secretary. Since his retirement from FDA, Hubbard has worked as a consultant. He received a master's degree in public policy from American University in 1974 and a bachelor's degree in History and American Studies from the University of North Carolina at Chapel Hill in 1970.

John K. Jenkins joined Greenleaf Health as Principal, Drug and Biological Products, following a distinguished 25-year career at the Food and Drug Administration (FDA). John received numerous awards from the FDA and external groups for his work on behalf of patients and his contributions to public health.

As Director of the Office of New Drugs (OND) at FDA's Center for Drug Evaluation and Research (CDER) from 2002 to 2017, Jenkins was responsible for more than 1,000 agency employees and 19 product review divisions. During that time, he oversaw the review of thousands of new drug applications and biological licensing applications as well as the approval of more than 400 new molecular entities. John was a critical figure in the development and implementation of programs under the Prescription Drug User Fee Act and implementation of the statutes and regulations that guide innovative drug development and regulation. Jenkins also played a leading role in implementing the biosimilar biologics

program in CDER under the Biologics Price Competition and Innovation Act and the Biosimilars User Fee Act, including approval of four biosimilar applications.

Jenkins began his FDA career in 1992, where he was a medical officer in the Division of Oncology and Pulmonary Drug Products. He subsequently served as Pulmonary Medical Group Leader and Acting Division Director before being appointed as Director of the Division of Pulmonary Drug Products in 1995. He became the Director of the Office of Drug Evaluation II in 1999 and remained in that position until he was appointed Director of OND in 2002.

Jenkins received his undergraduate degree in biology from East Tennessee State University in 1979 and his medical degree from the University of Tennessee at Memphis in 1983. He completed his postgraduate medical training in internal medicine, pulmonary disease, and critical care medicine at Virginia Commonwealth University/Medical College of Virginia (MCV) from 1983 until 1988.

Jenkins is Board Certified in internal medicine and pulmonary diseases by the American Board of Internal Medicine. Following completion of his medical training, John joined the faculty of MCV as an Assistant Professor of Pulmonary and Critical Care Medicine and as a Staff Physician at the Hunter Holmes McGuire VA Medical Center in Richmond, Virginia.

Aaron S. Kesselheim, M.D., J.D., M.P.H., is a Professor of Medicine at Harvard Medical School (HMS) and a faculty member in the Division of Pharmacoepidemiology and Pharmacoeconomics in the Department of Medicine at Brigham and Women's Hospital. Within the Division, Aaron created and leads the Program On Regulation, Therapeutics, And Law (PORTAL, www.PORTALresearch.org), an interdisciplinary research core focusing on intersections among prescription drugs and medical devices, patient health outcomes, and regulatory practices and the law. PORTAL is now among the largest, non-industry-funded academic centers focusing on these issues in the country (Twitter: @PORTAL_research, @akesselheim). Author of over 600 publications in the peer-reviewed medical and health policy literatures, Kesselheim has testified before Congress on pharmaceutical policy, medical device regulation, generic drugs, and modernizing clinical trials. He is a core faculty member at the HMS Center for Bioethics, where he co-teaches a course on health policy, law, and bioethics and organizes a monthly policy and ethics seminar series. Kesselheim also serves as a Visiting Professor of Law at Yale Law School and at the Yale School of Public Health, where he teaches yearly courses on Food and Drug Administration Law and Policy. He recently developed a massive open online course called *Prescription Drug Regulation, Cost, and Access: Current Controversies in Context* that has been disseminated via the HarvardX platform to over 100,000 participants world-wide. He is the editor-in-chief of the *Journal of Law, Medicine, and Ethics.* In 2020, he was elected to the National Academy of Medicine.

H. (Bert) G. Leufkens is Emeritus Professor of Regulatory science and pharmaceutical policy, Utrecht University, The Netherlands. Dr. Leufkens obtained his PharmD and Ph.D. degree from Utrecht University. In 1985 he was a Fulbright Fellow at the University of Minnesota. Dr. Leufkens has been research and policy-wise active at several (inter)national platforms on drug safety, pharmaceutical policy and regulatory science (e.g. past-member EMA Pharmacovigilance Working Party 2005-2009, chair of Dutch Medicines Evaluation Board (MEB) 2007-2017, past-member of the EMA CHMP 2009-2015, past-President of ISPE, 2008-2020 Scientific Director of the WHO Utrecht Collaborating Centre for Pharmaceutical Policy and Regulation).

Leufkens' research is inspired by an array of scientific and societal questions on the interface between medicines, pharma policy, innovation and regulation. Particularly his recent contributions to the surge of regulatory science work are well-acknowledged worldwide. The aim of the research is to gain deep insight, to elucidate dynamic policy and regulatory pathways, and to stimulate learning on why and how medicines are developed, regulated and used in clinical practice. Research topics include methods and

concepts of regulatory science, safety of biopharmaceuticals, orphan drugs, drug life-cycle dynamics and stakeholder involvement in decision making about approval and HTA of medicines.

Anabela Marçal is EMA (European Medicines Agency) Liaison Official to the U.S. Food and Drug Administration. She holds a degree in pharmacy and a professional certification in hospital pharmacy. Marçal had joined the EMA in 1999. During her career at the Agency, she held a number of roles in various areas, including Head of Committees and Inspections and Head of Clinical Trials.

Daniel O'Connor is a Leicester medical graduate with a background in cancer research, histopathology and oncology. His Ph.D. in the field of tumour suppressor genes was awarded from Imperial College London and he holds an M.Sc. in Oncology from the Institute of Cancer Research. He completed higher medical training in the UK in Pharmaceutical Medicine.

O'Connor joined the UK Medicines and Healthcare products Regulatory Agency (MHRA) as a Medical Assessor in 2006 from a clinical lecturer post at UCL/UCH/Ludwig Institute for Cancer Research. He is currently Deputy Director of the MHRA's Innovation Accelerator with special interests in rare diseases, early access, regulatory science, health innovation and patient reported outcomes and engagement.

He is editor author of the *Oxford Specialist Handbook of Pharmaceutical Medicine* and on the editorial board of *Expert Opinion on Orphan Drugs* and *Rare Disease and Orphan Drugs Journal*.

Pilar N. Ossorio, J.D., Ph.D., is Professor of Law and Bioethics at the University of Wisconsin, Madison, where she is on the faculties of the Law School and the Department of Medical History and Bioethics at the Medical School. In 2011 she became the inaugural Ethics Scholar-in-Residence at the Morgridge Institute for Research, the private, nonprofit research institute that is part of the Wisconsin Institutes of Discovery. She also serves as the co-director of UW's Law and Neuroscience Program, as a faculty member in the UW Masters in Biotechnology Studies program, and as Program Faculty in the Graduate Program in Population Health. Prior to taking her position at UW, she was Director of the Genetics Section of the Institute for Ethics at the American Medical Association, and taught as adjunct faculty at the University of Chicago Law School.

Ossorio received her Ph.D. in Microbiology and Immunology in 1990 from Stanford University. She went on to complete a post-doctoral fellowship in cell biology at Yale University School of Medicine. Throughout the 1990's Dr. Ossorio also worked as a consultant for the federal program on the Ethical, Legal, and Social Implications (ELSI) of the Human Genome Project, and in 1994 she took a full time position with the Department of Energy's ELSI program. In 1993 she served on the Ethics Working Group for President Clinton's Health Care Reform Task Force.

She received her J.D. from the University of California at Berkeley School of Law in 1997. While at Berkeley she was elected to the legal honor society Order of the Coif and received several awards for outstanding legal scholarship.

Steven D. Pearson, M.D., M.Sc., is the Founder and President of the Institute for Clinical and Economic Review (ICER). Pearson is also a Lecturer in the Department of Population Medicine at Harvard Medical School.

An internist, health services researcher, and ethicist, Pearson has served in many advisory and leadership roles in academia and government. In 2004 he was awarded an Atlantic Fellowship from the British Government and chose to serve as Senior Fellow at the National Institute for Health and Clinical Excellence (NICE). Returning to the United States in 2005 he was asked to serve during the George W. Bush Administration as Special Advisor, Technology and Coverage Policy, at the Centers for Medicare and Medicaid Services, and also accepted an appointment as Visiting Scientist in the Department of

Bioethics at the National Institutes of Health, a position he maintained from 2005-2019. In other roles, Pearson has also served as the Vice Chair of the Medicare Evidence Development and Coverage Advisory Committee (MedCAC).

Pearson's publications include over 150 peer-reviewed articles and commentaries on the role of evidence in the health care system, and the book *No Margin, No Mission: Health Care Organizations and the Quest for Ethical Excellence*, published by Oxford University Press.

Frank W. Rockhold,* Ph.D., is Professor of Biostatistics and Bioinformatics at the Duke University Medical Center, Faculty Director of Biostatistics at The Duke Clinical Research Institute, and Professor of Biostatistics at Virginia Commonwealth University. He has diverse research interests and consulting experience including clinical trial design, data monitoring committees, benefit/risk evaluation, and pharmacovigilance and has been a leader in the scientific community in promoting data disclosure and transparency in clinical research. His career includes numerous senior management positions in the pharmaceutical industry, including Senior Vice President of Biostatistics and Data Science and most recently as Chief Safety Officer and Senior Vice President for Pharmacovigilance at GSK. Rockhold has served as Chairman of the board for CDISC and is currently Chairman of the Board of the Frontier Science Research Foundation and an advisor to EMA. He serves on numerous Independent Data Monitoring Committees. He is a coauthor of the ICH (International Council for Harmonization) E9 (Biostatistics) and E10 (Control Group) guidelines.

Rockhold holds a B.A. in Statistics from The University of Connecticut, an ScM in Biostatistics from The Johns Hopkins University, and a Ph.D. in Biostatistics from the Medical College of Virginia at Virginia Commonwealth University. He is an Elected Fellow of the American Statistical Association, the Society for Clinical Trials, and the Royal Statistical Society. He is an Accredited Professional Statistician, PStat®, and a Chartered Statistician, CStat.

Joseph S. Ross, M.D., M.H.S., is a Professor of Medicine (General Medicine) and of Public Health (Health Policy and Management) at the Yale School of Medicine, an Associate Physician of the Center for Outcomes Research and Evaluation at Yale-New Haven Health System, and Co-Director of the National Clinician Scholars Program at Yale. With expertise in health services and outcomes research and in evaluating the translation of clinical research into practice, his research examines the use and delivery of higher quality care and addresses issues related to pharmaceutical and medical device regulation, evidence development, postmarket surveillance, and medical product adoption. Ross co-directs the Yale-Mayo Clinic Center for Excellence in Regulatory Science and Innovation (CERSI), the Yale Open Data Access (YODA) Project, and the Collaboration for Regulatory Rigor, Integrity, and Transparency (CRRIT) and leads efforts at Yale-New Haven Health System in collaboration with the National Evaluation System for health Technology (NEST). He has published more than 500 articles in peer-reviewed biomedical journals, co-founded the preprint server medRxiv, and is currently the U.S. Outreach and Research Editor at BMJ.

William B. Schultz** is a partner in the Washington, D.C. law firm Zuckerman Spaeder, where he specializes in Food and Drug and Healthcare Law. He served as General Counsel of the Department of Health and Human Services (2011-16), Deputy Commissioner of the Food and Drug Administration (1994-1998), and Deputy Assistant Attorney General, U.S. Department of Justice (1999-2000). He was also an attorney at Public Citizen Litigation Group for 14 years and counsel to the House Subcommittee on Health and Environment for five years. Schultz is a member of the National Academies of Sciences, Engineering, and Medicine's Committee on Science, Law and Technology and for 10 years he taught Food and Drug Law and Litigation at Georgetown University Law Center. He also is on the Leadership Council, Yale School of Public Health; Board of Trustees, Partners in Health; Board of Directors of the National Health Law Program; Board of Directors, Center for Science in the Public Interest; and Board of Directors, Historical Society of the D.C. Circuit. Schultz began his legal career clerking for Judge William

B. Bryant, U.S. District Court, D.C. He graduated from Yale College and the University of Virginia School of Law.

Joshua Sharfstein (NAM) is Vice Dean for Public Health Practice and Community Engagement and Professor of the Practice at the Johns Hopkins Bloomberg School of Public Health. He earned his M.D. from Harvard Medical School in 1996. He oversees the Office of Public Health Practice and Training and is director of the Bloomberg American Health Initiative. He also holds a faculty appointment in the Department of Health Policy and Management. Previously, he served as the Secretary of the Maryland Department of Health and Mental Hygiene, the Principal Deputy Commissioner of the U.S. Food and Drug Administration, as Commissioner of Health for Baltimore City, and as health policy advisor for Congressman Henry A. Waxman. He is the author of *The Public Health Crisis Survival Guide: Leadership and Management in Trying Times* (2018) and co-author of *The Opioid Epidemic: What Everyone Needs to Know* (2019), both from Oxford University Press.

Rachel E. Sherman, M.D., M.P.H., FACP, has more than 30 years of experience working in medical product development, evaluation, and approval of drugs and biosimilar products, combination products, and orphan drugs. During her career at the U.S. Food and Drug Administration (FDA), she worked closely with multiple medical product centers to implement key clinical, scientific, and regulatory programs, including expedited approval pathways; real world evidence generation and analysis; active postmarket surveillance; and expanded access. Until January 2019, Sherman served as Principal Deputy Commissioner, Food and Drugs at FDA.

An area of special interest to Sherman, spawned early in her career doing primary HIV care at the height of the epidemic, has been designing and developing approaches that help expedite medical product development and approval, including developing the accelerated approval standard. Ensuring that accelerated approval remains an option for patients with unmet needs remains a critical part of her work.

Today, Dr. Sherman is helping organizations to address regulatory challenges at all stages of product development. She is especially skilled at identifying and resolving potential premarket hurdles and assisting with post approval marketing, surveillance, and safety challenges.

A certified internist, Dr. Sherman has a specialty in infectious diseases.

Peter Stein, M.D., is the Director of the Center for Drug Evaluation and Research (CDER) at the U.S. Food and Drug Administration's Office of New Drugs (OND). OND is responsible for the regulatory oversight of investigational studies during drug development and decisions regarding marketing approval for new (innovator or non-generic) drugs, including decisions related to changes to already marketed products. OND provides guidance to regulated industry on a wide variety of clinical, scientific, and regulatory matters.

A nationally-recognized leader in pharmaceutical research and development, Stein joined CDER in 2016 as the OND Deputy Director. Before coming to FDA, he served as Vice President for late stage development, diabetes, and endocrinology at Merck Research Laboratories. He also served as Vice President, head of metabolism development at Janssen. He has more than 30 years of academic, clinical, and industry experience.

Stein holds a bachelor's degree in history from the University of Rochester in New York and a medical degree from University of Pennsylvania. He trained at Yale University and Yale-New Haven Hospital in internal medicine and in endocrinology and metabolism.

Harold Varmus, M.D., co-recipient of the 1989 Nobel Prize in Physiology or Medicine for studies of the genetic basis of cancer, joined the Meyer Cancer Center of Weill Cornell Medicine as the Lewis Thomas University Professor of Medicine in April 2015. He is also a Senior Associate Member of the New York Genome Center, where he helps to develop programs in cancer genomics. Previously, Dr. Varmus was the Director of the National Cancer Institute for five years, the President of Memorial Sloan-Kettering Cancer Center for 10 years, and Director of the National Institutes of Health for six years. A graduate of Amherst College and Harvard University in English literature and of Columbia University in medicine, he was further trained at Columbia University Medical Center, the National Institutes of Health, and the University of California San Francisco (UCSF), before becoming a member of the UCSF basic science faculty for over two decades. He is a member of the U.S. National Academies of Sciences and Medicine, is involved in several initiatives to promote science and health in developing countries, and serves on advisory groups for several academic, governmental, philanthropic, and commercial institutions. These positions currently include co-chair of the Mayor's LifeSciNYC initiative and member of advisory boards for Chan-Zuckerberg Science, the Broad and Crick Institutes, and three biotechnology companies (Surrozen, Dragonfly, and PetraPharma). The author of about 400 scientific papers and five books, including a 2009 memoir entitled *The Art and Politics of Science*, Varmus was a co-chair of President Obama's Council of Advisors on Science and Technology, a co-founder and Chairman of the Board of the Public Library of Science, and chair of the Scientific Board of the Gates Foundation Grand Challenges in Global Health.

***Member of Workshop Planning Committee**
****Co-chair of Workshop Planning Committee**