Processes to Evaluate the Safety and Efficacy of Drugs for Rare Diseases or Conditions in the United States and the European Union

Committee

Jeffrey Kahn

Chair

Dr. Jeffrey Kahn is the Andreas C. Dracopoulos Director of the Johns Hopkins Berman Institute of Bioethics, a position he assumed in July 2016. Since 2011, he has been the inaugural Robert Henry Levi and Ryda Hecht Levi Professor of Bioethics and Public Policy at the Berman Institute. He is also Professor in the Department of Health Policy and Management of the Johns Hopkins Bloomberg School of Public Health. He is an internationally recognized expert in bioethics, exploring the intersection of ethics and health/science policy, including human and animal research ethics, public health, and ethical issues in emerging biomedical technologies. Dr. Kahn has served on numerous governmental and international advisory panels, including most recently on the International Commission on the Clinical Use of Heritable Human Genome Editing. He is currently chair of the National Academies of Sciences, Engineering, and Medicine (National Academies) Committee on Aerospace Medicine and Medicine of Extreme Environments and has previously chaired the National Academies Committee on the Use of Chimpanzees in Biomedical and Behavioral Research (2011); the Committee on Ethics Principles and Guidelines for Health Standards for Long Duration and Exploration Spaceflights (2014); and the Committee on the Ethical, Social, and Policy Considerations of Mitochondrial Replacement Techniques (2016). Dr. Kahn has served as chair of the National Academy of Medicine Board on Health Sciences Policy and as a member of the National Academy of Medicine Council. He received his B.A. (microbiology) from the University of California, Los Angeles, his M.P.H. from Johns Hopkins University School of Hygiene and Public Health, and his Ph.D. (philosophy/bioethics) from Georgetown University.

Ronald Bartek

Member

Mr. Ronald Bartek is Co-founder and President of Friedreich's Ataxia Research Alliance (FARA). Formerly, he was Director & Chair of the National Organization for Rare Disorders. He also served as past President and current member of the board of directors of the Alliance for a Stronger FDA. Mr. Bartek co-founded the NCATS Alliance, now serving as chair of its board of directors. He was formerly on the board of directors of the Alliance for Regenerative Medicine. He is a member of the NIH/NCATS National Advisory Council and the NIH Neurological Institute National Advisory Council. Mr. Bartek has previously served as chair of the NCATS Cures Acceleration Network Review Board. Mr. Bartek has been recognized by FDA Office of Orphan Drug Development as one of “30 Heroes changing lives of rare disease patients”. He is a former member of the FDA/CTTI Patient Engagement Collaborative. Mr. Bartek has twenty years of federal executive and legislative experience in defense, foreign policy and intelligence. Mr. Bartek received his B.S. from U.S. Military Academy, West Point and his M.A. from Georgetown University.

Terry Jo Bichell

Member

Dr. Terry Jo Vetters Bichell is the CEO and founder of COMBINEDBrain as well as a Lecturer at Vanderbilt University. She worked primarily as a public health nurse-midwife until her youngest child, Lou, was diagnosed with Angelman syndrome in 2000. She quickly switched focus to move bench research into the first clinical trials for Angelman syndrome and to help design natural history studies. Dr. Bichell was the founding director of the A-BOM Alliance from 2016-2018. In 2019, she launched COMBINEDBrain, a pre-competitive consortium of patient advocacy organizations that work with clinicians, researchers, and pharmaceutical firms to identify outcome measures and biomarkers for rare genetic neurodevelopmental disorders. She is the Vice Chair of the Tennessee Rare Disease Advisory Council and teaches a course in translational neuroscience at Vanderbilt University. She previously served on the Angelman Patient Advisory Council to Hoffman La Roche, which has now been paused. As a parent of a child living with a rare disease, Terry Jo gained first-hand experience in the search for a treatment for Angelman syndrome as she accompanies her son, Lou, as he participates in clinical trials. Dr. Bichell received her B.S.N. from St. Louis University, her M.P.H. from Boston University, and her Ph.D. in neuroscience from Vanderbilt University where she studied gene-environment interactions in Huntington’s disease rodent models.

Edward A. Botchwey

Member

Dr. Edward Botchwey is a Professor in the Wallace H. Coulter Department of Biomedical Engineering at Georgia Tech and Emory University. His research focuses on elucidating lipid signaling mechanisms in sickle cell disease and developing novel immunomodulatory therapies to resolve inflammation and treat organ dysfunction. Dr. Botchwey has garnered over $14 million in research funding and has published extensively on bioactive lipids and resolution pharmacology for sickle cell therapies. He conducts research in affiliation with the Marcus Center for Therapeutic Cell Characterization and Manufacturing (MC3M). He has also spearheaded efforts to increase diversity and inclusion in biomedical engineering, including leading the Diversity, Equity, and Inclusion Committee for the Society for Biomaterials. Dr. Botchwey received his B.S. from the University of Maryland and his MEng and Ph.D. from the University of Pennsylvania. He completed a postdoctoral fellowship in Vascular Biology at The Wistar Institute.

Shein-Chung Chow

Member

Dr. Shein-Chung Chow is a Professor at the Department of Biostatistics and Bioinformatics at Duke University School of Medicine. Dr. Chow is also an Adjunct Professor at Peking University and Beijing Capital Medical University. Previously, he was a special government employee (SGE), appointed by the FDA as a voting member of the Oncologic Drug Advisory Committee (ODAC) and a Statistical Advisor to the FDA. Dr. Chow previously served as Associate Director of the Center for Drug Evaluation and Research’s Office of Biostatistics at the FDA. Dr. Chow is currently serving on several Data Safety Monitoring Boards for clinical studies sponsored by Genetech and Merck via third party clinical research organizations, Syneos, Parexel, and Statistics Collaborative. Dr. Chow was the Editor-in-Chief of the Journal of Biopharmaceutical Statistics (1992-2020) and is the Editor-in-Chief of the Biostatistics Book Series at Chapman and Hall/CRC Press of Taylor & Francis Group. He was elected Fellow of the American Statistical Association in 1995 and elected member of the ISI (International Statistical Institute) in 1999. Dr. Chow is the author or co-author of over 200 papers and 20 books on trial design considerations and statistical methods for rare disease research. He more recently authored the book, “Innovative Methods for Rare Disease Drug Development” Dr. Chow received his B.S. from National Taiwan University and his Ph.D. from University of Wisconsin, Madison.

Hans-Georg Eichler

Member

Dr. Hans-Georg Eichler is the Consulting Physician at the Association of Austrian Social Insurances. Prior to this role, Dr. Eichler was the Senior Medical Officer of the European Medicines Agency (EMA) from 2007-2021. Earlier in his career, Dr. Eichler was a Professor of Clinical Pharmacology and Head of the Department of Clinical Pharmacology, as well as Vice-Rector for Research and International Relations at the Medical University of Vienna. During his time in academia, he gained ample experience in clinical research, including being the primary investigator of numerous academic and industry-sponsored drug trials. In his subsequent positions, he was closely involved in the development of pharmaceutical policies from the governmental, regulatory and public payer perspectives. Dr. Eichler serve as the vice-chair of the Scientific Advisory Committee at the Centre for Innovation in Regulatory Sciences and has done pro bono work for EURORDIS-Rare Diseases Europe, a non-profit alliance of over 1000 rare disease patient organizations across Europe. Dr. Eichler has received several honors and awards from different European universities and learned societies. He received his M.Sc. from the University of Surrey and his M.D. from the University of Vienna.

Pat Furlong

Member

Ms. Pat Furlong is President and CEO of Parent Project Muscular Dystrophy (PPMD), which focuses on Duchenne and Becker muscular dystrophy. She led the development of Draft Guidance on Duchenne, which was submitted to FDA in 2016 and updated in 2022. She serves as a member for the Duchenne Community Advisory Board in Europe. Ms. Furlong served on the National Academies of Sciences, Engineering, and Medicine (National Academies) Committee on Safe and Effective Medicines for Children. Ms. Furlong is a nurse practitioner by training, spending her early career in whole organ transplantation and renal dialysis. Since 1994, Ms. Furlong has focused on rare disease research - specifically on genetic testing, standards of care, therapy development, and regulatory processes. She has led preference studies to understand the patient’s perspective of benefit and risk. Ms. Furlong has worked with rare disease groups as they prepare for interactions with regulatory agencies in the U.S.A. and Europe. Ms. Furlong is a member of the World Duchenne Organization’s board, data safety monitoring boards for the Rare Disease Research Network and Cooperative International Neuromuscular Research Group, and NYU’s Pediatric Gene Therapy Medical Ethics Group. Ms. Furlong serves on the Clinical Trials Transformation Initiative (CTTI) executive committee and is a board member with the National Health Council. She received her R.N./B.S.N. degree from Mount St. Joseph University.

Steven K. Galson

Member

Dr. Steven Galson is a senior advisor to Boston Consulting Group and serves on the board of directors of Biocryst Pharmaceuticals and Elephas biosciences. He is a consultant for Skyline Therapeutics. Until June 2020, Dr. Galson was a Senior Vice President of Research and Development at Amgen Inc. He spent more than 20 years in government service, including two years as Acting Surgeon General of the United States. He served as Director of the FDA's Center for Drug Evaluation and Research, where he provided leadership for the center's national and international programs in pharmaceutical regulation. Dr. Galson began his public health service career as an epidemiological investigator at the U.S. Centers For Disease Control and Prevention. He was also the Chief Medical Officer at both the Environmental Protection Agency and the U.S. Department of Energy. Dr. Galson is a trustee of the Keck Graduate Institute and a member of the Executive Committee of the Clinical Trial Transformation Initiative. In 2008, he received an honorary Doctor of Public Service degree from Drexel University School of Public Health, and in 2015, he received the Jacobi Medallion Award from Icahn Mount Sinai School of Medicine. In 2018 he was named health leader of the year from the Commissioned Officers Association of the U.S. Public Health Service. Dr. Galson has been a member of the Forum on Drug Discovery Development and Translation of the National Academies of Sciences, Engineering, and Medicine (National Academies) for 20 years. He has also served two terms as the Forum's co-chair. Dr. Galson received his B.S. from Stony Brook University, his M.P.H. from Harvard University, and his M.D. from Icahn Mount Sinai School of Medicine.

Gavin Huntley-Fenner

Member

Dr. Gavin Huntley-Fenner is the co-founder and Principal Human Factors Consultant at Huntley-Fenner Advisors, which provides scientific advisory services. Specializing in creative and scientifically based approaches to assessing risks and benefits, Dr. Huntley-Fenner brings over 25 years of both academic and business experience to bear on the crafting of innovative and effective communication to consumers. Dr. Huntley-Fenner also serves as an expert legal consultant, public speaker, and facilitator of risk analysis teams, where he is noted for his ability to effectively articulate solutions to complex problems. He has served on the U.S. FDA Risk Communication Advisory Committee (2004-2009) and on the National Academies of Sciences Engineering and Medicine (NASEM) Mutual Recognition Agreements in the Regulation of Medicines Study Committee (2019-20). He is currently a member of the NASEM Environmental Health Matters Initiative Steering Committee and is also a member of Mattel's Medical and Scientific Advisory Council. Dr. Huntley-Fenner received his Ph.D. in Brain and Cognitive Sciences from Massachusetts Institute of Technology in 1995 and his B.A. in Cognitive Sciences from Vassar College in 1990. Edits made to the bio of Gavin Huntley-Fenner on August 19, 2024.

Anaeze C. Offodile, II

Member

Dr. Anaeze C. Offodile II is an Executive Vice President and the Chief Strategy Officer of Memorial Sloan Kettering Cancer Center (MSK). Dr. Offodile is a member of the Forum on Drug Discovery Development and Translation of the National Academies of Sciences, Engineering, and Medicine (National Academies). He is a double board-certified physician with clinical expertise in oncologic reconstruction, a health services researcher with a focus on alternative payment models and care redesign, and a healthcare administrator with management experience in academia. He leads strategy efforts and care transformation initiatives at MSK by continuing to develop the core infrastructure, management systems, and processes for enterprise strategy and business development. Dr. Offodile pilots new initiatives, facilitates alignment on strategic institutional priorities, leverages data sources to cultivate innovative digital analytics and products, develops collaborations with outside groups, and partners with key internal leaders to competitively position MSK for the future. He received his B.S. from Kent State University, his M.P.H. from Johns Hopkins Bloomberg School of Public Health, and his M.D. from Columbia University.

Anne Pariser

Member

Dr. Anne Pariser is a physician, currently working with the Indian Health Service at the Crow/Northern Cheyenne Hospital in Crow Agency, Montana. Prior to this, she was the VP, Medical and Regulatory Affairs at Alltrna, a biotech company developing tRNAs as therapeutic agents for rare genetic diseases (through March 2024), where she continues to provide part-time consulting services. Previously, she was the Director of the Office of Rare Diseases Research at the NIH National Center for Advancing Translational Sciences from 2017-2022. From 2000-2017, she worked at the FDA CDER Office of New Drugs, where she led the first specialized rare diseases review team for Inborn Errors of Metabolism. In 2010, Dr. Pariser founded the Rare Diseases Program (RDP) within OND/CDER, a Congressional Mandate intended to accelerate and improve rare diseases drug review within FDA that focused on the development of policy, guidance, training, and coordination of regulatory science to benefit rare disease programs. Dr. Pariser’s research has focused on rare diseases regulatory and translational sciences, and she is the author of approximately 50 papers on these topics. Dr. Pariser has received numerous awards from FDA, NIH, HHS and other stakeholders for her service to the rare disease community. This includes the National Organization for Rare Disorders’ National Public Health Leadership Award and being named, along with the OND/CDER’s Rare Diseases Program, as one of the 30 Rare Disease Heroes on the 30th anniversary of the Orphan Drug Act. She is the chair of the Regulatory Scientific Committee at the International Rare Disease Research Consortium, a collaborative initiative uniting national and international non-profits, industry, patient advocacy organizations, and scientists to promote collaboration for rare disease research. She is also a volunteer member of the Board of Directors for the Undiagnosed Diseases Network Foundation (UDNF) – a non-profit that fosters collaboration among patients, clinicians, and scientists to bridge diagnosis, research, and clinical care for undiagnosed patients with rare and ultra-rare diseases. Dr. Pariser received her B.S. from Bates College and her M.D. from Georgetown University School of Medicine. She is board certified in Internal Medicine. Edits made to the bio and unavoidable conflict of interest statement of Anne Pariser on May 17, 2024.

Jonathan H. Watanabe

Member

Dr. Jonathan H. Watanabe is Associate Dean of Assessment and Quality at the UC Irvine School of Pharmacy & Pharmaceutical Sciences and Director of the UCI Center for Data-Driven Drugs Research and Policy. Dr. Watanabe is a member of the Forum on Drug Discovery Development and Translation of the National Academies of Sciences, Engineering, and Medicine (National Academies). Dr. Watanabe is an appointed member of the non-partisan California Health Benefits Review Program Faculty Task Force of the University of California funded by the State of California Legislature. He has been involved in research and policy efforts salient to rare diseases, including service on the National Academies' Committee on Making Medicines Affordable and participation in the National Academies' workshop series on Examining the Impact of Real-World Evidence on Medical Product Development. The latter also entailed publication of guidance manuscripts on conducting real-world trials in special populations. Additionally, he has received grant approval for a pending GlaxoSmithKline-funded project that will use publicly available data to examine global definitions of what deems drug as a 'biosimilar'. He has published original research examining the increase in high-spend medications covered by Medicare Part D that serve small populations in the U.S. He was the National Academy of Medicine (NAM) Anniversary Fellow in Pharmacy from 2016 to 2018. From 2018 to 2021, he served as a Scholar in the NAM Emerging Leaders in Health and Medicine Program. Dr. Watanabe was the inaugural recipient of the University of Washington/Allergan Global Health Economics and Outcomes Research Fellowship (2007 to 2009). He received his B.S. from the University of Washington, his Pharm.D. from the University of Southern California, and his M.S. and Ph.D. from the University of Washington Comparative Health Outcomes, Policy, and Economics (CHOICE) Institute. He is a board-certified geriatrics pharmacist.