

Translational Science in Drug Development: Surrogate Endpoints, Biomarkers, and More

Virtual Public Workshop

May 24, 2022 | 12:00 pm – 4:00 pm ET

May 25, 2022 | 12:00 pm – 4:00 pm ET

Biographies



Jeff Allen serves as the President and CEO of Friends of Cancer Research (*Friends*). For over 25 years, *Friends* has created unique scientific partnerships, accelerated policy change, and supported groundbreaking research to deliver new therapies to patients quickly and safely. As a key thought leader on issues related to the U.S. Food and Drug Administration, healthcare, and regulatory policy, he is regularly published in prestigious medical journals and policy publications and has contributed his expertise to the legislative process on multiple occasions. Recent *Friends* initiatives include the establishment of the Breakthrough Therapies designation, innovative research consortia to enhance biomarker development, and the launch of a unique cross-sector partnership to accelerate clinical trial conduct and rapidly assess if a patient's treatment is working. Jeff received his Ph.D. in cell and molecular biology from Georgetown University and holds a Bachelor of Science in Biology from Bowling Green State University.



Patrick Archdeacon is the Associate Director for Therapeutics in the Division of Diabetes, Lipid Disorders, and Obesity (DDLO) in the Office of New Drugs (OND) in the Center for Drug Evaluation and Research (CDER) at the Food and Drug Administration (FDA). Dr Archdeacon originally joined FDA in 2008 as a medical officer in the Division of Special Pathogens and Transplant Products in the Office of New Drugs. He attended medical school at Columbia University's School of Physicians and Surgeons. Prior to joining FDA, he completed his training in internal medicine at the New York Presbyterian Hospital and in nephrology and transplant nephrology at the University of North Carolina.



Issam Awad is the John Harper Seeley Professor of Neurological Sciences, Professor of Neurological Surgery, Neurology, the Grossman Institute of Neuroscience, Quantitative Biology and Human Behavior, and the Committee on Neurobiology at the University of Chicago Medicine and Biological Sciences. He is also the Director of Neurovascular Surgery, a Senior Faculty Scholar of the Bucksbaum Institute for Clinical Excellence, and the Director of the Safadi Program of Excellence in Clinical and Translational Neurosciences. Dr. Awad was born in Lebanon and pursued his university studies in the United States, including BS, MSc, and MD degrees from Loma Linda University in California, neurosurgery residency at the Cleveland Clinic and neurovascular fellowship at the Barrow Neurological Institute. He has worked in academia for more than 35 years, holding endowed or tenured professorships at Yale, Colorado and Northwestern before relocating 12 years ago to the University of Chicago. He is widely recognized for career long contributions and special skills in

Neurovascular Surgery. He has made numerous scientific discoveries including the characterization of subcortical ischemic lesions in the aged, advances in the understanding of the natural history and biologic behavior of vascular malformations of the brain, and technical developments in neurovascular and epilepsy surgery. His research has been funded by the U.S. National Institutes of Health since 1998, focusing currently on molecular mechanisms of cerebral vascular malformations and their biomarkers and therapeutic targets, and minimally invasive surgical techniques for hemorrhagic stroke. Awad's innovations are changing neurosurgical practice, and he has trained scores of neurosurgeons, some of whom are leading neurosurgical departments in a number of countries. His scholarly work has been cited more than 80,000 times (H-Index 90). Dr. Awad has served in leadership roles at several national professional societies, including as President of the Congress of Neurological Surgeons, Chairman of the Joint Cerebrovascular Section of the American Association of Neurological Surgeons and Congress of Neurological Surgeons, Governor of the American College of Surgeons, and the Executive Committee of the American Stroke Association. He was elected to the American Academy of Neurological Surgeons and the Society of Neurological Surgeons, and was inducted in 2018 to the Association of American Physicians. He served from 2016 to 2020 on the U.S. National Advisory Council for Neurological Disorders and Stroke.



Anthony G. Durmowicz is Vice President of Clinical Development for the Cystic Fibrosis Foundation (CFF) headquartered in Bethesda where he helps lead CFF drug development activities across pulmonary, gastrointestinal, antimicrobial, and anti-inflammatory areas, acts as a CFF regulatory consultant, and contributes to development of the Foundation's scientific strategy and priorities. Prior to CFF he was a medical officer and clinical team leader at the FDA's Division of Pulmonary, Allergy, and Rheumatology Products (DPAAP) for 12 years. Prior experience also includes work as Director, Clinical Development, Respiratory Diseases at MedImmune (now Astra Zeneca) primarily focused on developing therapies for their monoclonal antibody programs for severe asthma and respiratory syncytial viral infection. Dr. Durmowicz had a 20-year career in academic medicine prior to his industry and non-profit pursuits. He received his B.A. from Loyola University (Maryland) and M.D. from the University of Maryland. He did his pediatric residency and fellowships in pediatric pulmonology and pediatric critical care at the University of Colorado and Denver Children's Hospital. His academic career included conducting basic scientist research studying pulmonary vascular development and pediatric pulmonary hypertension and the effects of hypoxia and high altitude on lung vascular structure and function and as a pediatric critical care clinician. Dr. Durmowicz was Director of the Pediatric ICU at Denver Children's Hospital and of the Pediatric Critical Care Fellowship at University of Colorado. He has had research and clinical appointments at the Universities of Colorado and Utah, Washington University in St. Louis, and Johns Hopkins University.



Jennifer Farmer is the Chief Executive Officer of the Friedreich's Ataxia Research Alliance. Jennifer has a Master's degree in Genetic Counseling and prior to joining FARA she worked at the University of Pennsylvania and Children's Hospital of Philadelphia. As a genetic counselor, Jennifer developed a special interest in neurogenetic conditions and then went on to establish and coordinate clinical and research programs for individuals and families diagnosed with Friedreich Ataxia (FA) and other neurodegenerative diseases. Having established relationships with the families who founded FARA and sharing in their vision to treat and cure FA she joined the organization full time in 2006. Jennifer has led FARA's efforts to establish clinical research

infrastructure and clinical trial readiness, grown the research grant program from funding <1M annually to >6M annually, led efforts to engage bio-pharma industry in FA drug discovery and development, and ensured highly efficient and transparent organizational growth and development. In her current role at FARA as CEO, she helps to carry out the strategic mission of the organization through leading FARA's research and partnership initiatives.



Christine Garnett is a clinical reviewer and team leader in the Division of Cardiology and Nephrology. She leads CDER's interdisciplinary review team for cardiac safety studies. Since 2008, Dr. Garnett has represented the FDA in the International Council for Harmonisation for the E14 guideline, and currently serves as FDA's Topic Leader. For her work on the ICH E14 guideline and advancing science in cardiac safety, she received ASCPT's Gary Neil prize for innovation in drug development in 2019. Dr. Garnett has published over 40 manuscripts in peer-reviewed journals in the area of cardiac safety, and received both Critical Path and Office of Women's Health grants.

Prior to joining the Division of Cardiology and Nephrology, she was a Team Leader and Associate Director of Operations in the Division of Pharmacometrics in the Office of Clinical Pharmacology. She obtained her PharmD degree from the University of Maryland and completed her clinical pharmacology fellowship with a focus in pharmacometrics and regulatory science at Georgetown University.



Leslie Gordon is a co-founder of The Progeria Research Foundation and serves as the organization's volunteer Medical Director. Dr. Gordon is the Principal Investigator for ongoing PRF programs for Progeria, including the PRF International Progeria Registry, Medical and Research Database, Cell and Tissue Bank, and the Genetic Diagnostics Program. She has organized 8 National Institutes of Health-funded, international scientific meetings on Progeria. She is Professor of Pediatrics Research at Hasbro Children's Hospital and the Alpert Medical School of Brown University in Providence, RI; Research Associate in Anesthesia at Harvard Medical School and Boston Children's

Hospital and Research Scientist at Women & Infants Hospital, all in Boston, MA. She was co-author on the 2003 gene discovery for Progeria, lead author of the 2012 Progeria treatment discovery study, and is co-chair of four Progeria clinical drug trials at Boston Children's Hospital. She has received the March of Dimes Basil O'Connor Award, the American Heart Association Scientist Development Award, The Gerontological Society of America Award for contributions to Progeria, a National Institutes of Health Bench to Bedside Grant, and the Mother of the Year award from Working Mother Magazine. Dr. Gordon earned her bachelor's degree in Zoology from the University of New Hampshire in Durham, New Hampshire. She went on to receive a master's degree and medical and doctorate degrees from Brown University and the Brown University School of Medicine respectively, where she achieved top honors in the medical program.



Nicole Gormley is the Division Director for the Division of Hematologic Malignancies II at the U.S. Food and Drug Administration. Dr. Gormley joined the FDA in 2011 and previously served as a clinical reviewer and the Multiple Myeloma Clinical Team Lead. While in these roles, Dr. Gormley has actively engaged with the multiple myeloma community on the development of novel endpoints, including minimal residual disease, and methods to address racial disparities. Dr. Gormley completed fellowship training in hematology and critical care at the National Institutes of Health and served

as the Deputy Clinical Director at the National Heart, Lung and Blood Institute prior to joining the Food and Drug Administration.



Steve Hoffmann is an Associate Vice President in Research Partnerships and Director of the Biomarkers Consortium at the Foundation for the National Institutes of Health (FNIH). He provides strategic planning, programmatic management and research administration of a multi-faceted portfolio of established and emerging projects within the Biomarkers Consortium and Accelerating Medicines Partnership including projects including neuroscience, rare diseases, organ toxicity, infectious disease and other autoimmune and inflammatory diseases.

Steve has a broad background in the academic, government and industry sectors in the field of translational biomarkers, molecular immunology and precision medicine. Prior to joining FNIH, Steve worked as both a project and product manager, supporting protein diagnostics development at Meso Scale Discovery (MSD). Before MSD, Steve was a scientist in the Transplantation and Autoimmunity Branch of the National Institute of Diabetes & Digestive & Kidney Diseases (NIDDK) and led research efforts utilizing immune depletion and co-stimulatory blockade strategies for human renal and islet cell transplant trials. Steve holds a Master of Science degree in Pathology and Laboratory Medicine from the University of North Carolina at Chapel Hill and a Bachelor of Science degree in Biochemistry and Biophysics from the University of Pittsburgh.



Lesley Inker is a Professor of Medicine at Tufts University School of Medicine (TUSM), an attending physician in the William B. Schwartz, MD Division of Nephrology at Tufts Medical Center (MC), and Medical Director of the Kidney And Blood Pressure Center at Tufts MC. Dr. Inker's primary research interests are in kidney function measurement and estimation, alternative endpoints for clinical trials of kidney disease progression, and epidemiology and outcomes related to CKD. Dr. Inker is an investigator on several trials of kidney disease progression. Dr Inker has worked with National Kidney

Foundation (NKF) leadership on multiple public health initiatives for CKD care in the United States. Dr. Inker is the inaugural chair of the steering committee for the NKF Patient Network, the first national kidney disease patient registry. She recently joined the Medical Advisory Board for the Alport's syndrome.

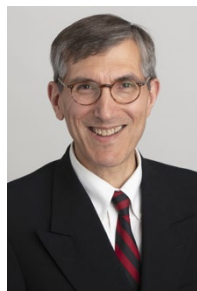


Kerry Jo Lee is the Associate Director for Rare Diseases in the Division of Rare Diseases and Medical Genetics, Office of New Drugs (OND), Center for Drug Evaluation and Research (CDER). In this role she leads the Rare Diseases Team, a multidisciplinary rare disease programming and policy team that works to promote their mission to facilitate, support, and accelerate the development of drugs and therapeutic biologics for rare diseases. Dr. Lee joined the FDA as a medical officer in 2014 with the former Division of Gastroenterology and Inborn Errors Products, OND, CDER. Dr. Lee then moved to a position as a clinical advisor for the Office of New Drug Policy, CDER,

where she served as a lead in the areas of benefit-risk assessment, modernization efforts (including the integrated review for marketing applications), and real-world data/evidence programming before serving in her current position.

Dr. Lee is a pediatric gastroenterologist/hepatologist. She is a graduate of Princeton University and the New York University School of Medicine with an honors degree conferred in microbiology. She completed her residency in pediatrics at the Children's Hospital of Los Angeles followed by a post-

doctoral clinical fellowship in Pediatric Gastroenterology, Hepatology, and Nutrition at Columbia University College of Physicians and Surgeons in New York. Dr. Lee maintains a steadfast interest in international policy and bioethics and worked for several years at the former National Bioethics Advisory Commission on reports advising the executive branch on ethical and policy issues in both international and domestic clinical trials.



Peter Marks is Director of the Center for Biologics Evaluation and Research (CBER) at FDA. He received his graduate and medical degrees from New York University. Following this, he completed an Internal Medicine residency and Hematology/Medical Oncology fellowship at Brigham and Women's Hospital in Boston, where he subsequently joined the attending staff as a clinician-scientist and eventually served as Clinical Director of Hematology. He then moved on to work for several years in the pharmaceutical industry prior to returning to academic medicine at Yale University where he served as Chief Clinical Officer of Smilow Cancer Hospital. He joined the FDA in 2012 as Deputy Center Director for CBER and became Center Director in January 2016.



Estelle Marrer-Berger is a senior Translational Safety Leader at Roche. She brings nearly 20 years' of experience in drug development, from early to late stage, across various indications and modalities. She is recognized for her expertise in translational development, in particular for her leadership pioneering and applying New Approach Methodologies (NAMs) to support Entry into Human milestones. As such, she has led multiple IND applications that leveraged a NAM-only approach using an integrated patient-centric framework. Dr. Marrer-Berger began her career at Novartis, where she held positions of increasing responsibility within the Biomarker Development, Investigative Toxicology and Preclinical Safety functions. She earned a Ph.D. in Microbiology and a Bachelor of Science degree in Cellular and Molecular Biology from the Université Louis Pasteur in Strasbourg, France.



Michael Pacanowski is the Director of the Division of Translational and Precision Medicine in FDA's Office of Clinical Pharmacology. He oversees a multidisciplinary team of clinical scientists who lead the Office's regulatory review, research, and policy activities related to pharmacogenomics, biomarker development, targeted therapies, and rare diseases. Dr. Pacanowski received his Pharm.D. from the Philadelphia College of Pharmacy and his M.P.H. from the University of Florida. He completed a residency in clinical pharmacology at Bassett Healthcare in Cooperstown, NY, and a clinical research fellowship in cardiovascular pharmacogenomics at the University of Florida.



Terina N. Martinez is Executive Director of the Duchenne Regulatory Science Consortium (D-RSC) and the Critical Path to Therapeutics for the Ataxias (CPTA) at the Critical Path Institute (C-Path). Terina received her undergraduate degree in Biology from the University of Dallas and earned a Ph.D. in Integrative Biology from the University of Texas Southwestern Medical Center at Dallas, where she studied cellular and molecular neuroscience. She completed her postdoctoral training at The University of Pittsburgh. Prior to joining C-Path, Terina was a Senior Associate Director, Research Programs at The Michael J. Fox Foundation for Parkinson's

Research in New York, New York and an application and collaboration scientist with Taconic Biosciences based in Cambridge, MA.

In her current role, Terina leads initiatives to build better drug development tools for Duchenne muscular dystrophy and inherited ataxias by convening a collaborative and consensus-driven community of diverse stakeholders including patient advocates, clinicians, researchers, pharmaceutical industry members, and regulatory agencies.



Mark McClellan is Director and Robert J. Margolis, M.D., Professor of Business, Medicine and Policy at the Margolis Center for Health Policy at Duke University. He is a physician-economist who focuses on quality and value in health care, including payment reform, real-world evidence and more effective drug and device innovation. Dr. McClellan is at the center of the nation's efforts to combat the pandemic, the author of COVID-19 response roadmap, and co-author of a comprehensive set of papers and commentaries that address health policy strategies for COVID vaccines, testing, and treatments, nationally and globally. He is former administrator of the

Centers for Medicare & Medicaid Services and former commissioner of the U.S. Food and Drug Administration, where he developed and implemented major reforms in health policy. Dr. McClellan is an independent board member on the boards of Johnson & Johnson, Cigna, Alignment Healthcare, and PrognomiQ; co-chairs the Guiding Committee for the Health Care Payment Learning and Action Network; and serves as an advisor for Arsenal Capital Group, Blackstone Life Sciences, and MITRE.



David M. Reese is executive vice president, Research and Development. In this role, Dr. Reese oversees Discovery Research, Global Development, Global Regulatory Affairs and Safety, as well as Global Medical. Dr. Reese joined Amgen in 2005 and has served in various leadership roles within the Research and Development organization. This includes most recently serving as Senior Vice President of Translational Sciences and Oncology where he oversaw the translation of Amgen's medicines from the lab into the clinic and the overall oncology strategy.

Prior to joining Amgen, Dr. Reese was director of Clinical Research for the Breast Cancer International Research Group (BCIRG) and a co-founder, president and chief medical officer of Translational Oncology Research International (TORI), a not-for-profit academic clinical research organization. Dr. Reese is a graduate of Harvard College and the University of Cincinnati College of Medicine. He completed training in Internal Medicine and Hematology/Oncology at the University of California, Los Angeles (UCLA) School of Medicine, and subsequently served on the faculty at UCLA and the University of California, San Francisco.



Joni L. Rutter is the acting director of the National Center for Advancing Translational Sciences (NCATS) at the National Institutes of Health (NIH). She oversees the planning and execution of the Center's complex, multifaceted programs that aim to overcome scientific and operational barriers impeding the development and delivery of new treatments and other health solutions. Under her direction, NCATS supports innovative tools and strategies to make each step in the translational process more effective and efficient, thus speeding research across a range of diseases, with a particular focus on rare diseases. By advancing the science of translation, NCATS helps

turn promising research discoveries into real-world applications that improve people's health. In her prior role as the NCATS deputy director, Rutter collaborated with colleagues from government, academia, industry, and nonprofit patient organizations to establish robust interactions with NCATS programs.

Prior to joining NCATS, Dr. Rutter served as the director of scientific programs within the *All of Us* Research Program, where she led the scientific programmatic development and implementation efforts to build a national research cohort of 1 million or more U.S. participants to advance precision medicine. While at NIH, she also led the Division of Neuroscience and Behavior at the National Institute on Drug Abuse (NIDA). In this role, she developed and coordinated research on basic and clinical neuroscience, brain and behavioral development, genetics, epigenetics, computational neuroscience, bioinformatics, and drug discovery. Rutter also coordinated the NIDA Genetics Consortium and biospecimen repository.

During her career, Dr. Rutter has earned a national and international reputation for her diverse and unique expertise via her journal publications and has received several scientific achievement awards, including a SmithKline Beecham Student Award in Pharmacology, a Janssen Research Foundation Young Investigator Award, and a Fellowship Achievement Award from the National Cancer Institute.

Rutter received her doctorate from the Department of Pharmacology and Toxicology, Dartmouth Medical School, Hanover, New Hampshire, and completed a fellowship at the National Cancer Institute within the Division of Cancer Epidemiology and Genetics.



Steve Ryder is a senior pharmaceutical R&D executive with broad experience at the highest levels of global development leadership. During the past 35+ years, he has been centrally involved in the global development of many products. In January 2019 he joined the team at Rallybio, a biotechnology company dedicated to transforming the lives of patients with devastating rare and ultra-rare disease. Prior to this he was SVP, Chief Development Officer at Alexion Pharmaceuticals (2013- 2018) where responsibilities included the global development, registration, and approval of new biological/drug candidates and building the organization that sustains these activities,

and founding President, Astellas Pharma Global Development (2008-2013) leading an organization of over 1000 staff in Japan (Tokyo), the US (Northbrook IL), and the EU ((Leiden, Netherlands) and reporting directly to the global CEO of Astellas Pharma, Inc. Recent approvals include Ultomiris™ for patients with Paroxysmal Nocturnal Hemoglobinuria (US '18), Soliris™ for patients with refractory generalized Myasthenia Gravis (EU/US/Japan '17), Strensiq™ for the ultra-rare disease of hypophosphatasia (Japan/US/EU '15), and Betanis/Myrbetriq™ for OAB (Japan '11, US/EU '12), the first beta-3 agonist approved for any indication. He is a member of the Board of Directors of Levo Therapeutics. He is past-President of the American Society of Clinical Pharmacology and Therapeutics, has held leadership positions in the Health Section Governing Board, Regulatory Executive Committee, and Board of BIO and the Science and Regulatory section of PhRMA, is a member of the Clinical

Pharmacology Advisory Board of the PhRMA Foundation and several editorial boards. He has served as the industry representative on FDA's Metabolic/Endocrine Drugs Advisory Committee and is past-Chairman of the Board of Directors of Gaylord Hospital (Wallingford, CT).



Oleg Shchelochkov is a board-certified pediatrician, clinical geneticist and medical biochemical geneticist, and is currently an associate investigator at NHGRI. After his early pediatrics training at the University of Iowa and genetics training at Baylor College of Medicine, Dr. Shchelochkov was a tenure-track assistant professor within the Pediatrics Department and Division of Genetics at the University of Iowa Hospitals and Clinics. He has been part of the NHGRI research community since 2015, starting as a staff clinician. Until recently, he was an associate research-physician studying many aspects of organic acidemias. In 2021, NHGRI appointed Dr. Shchelochkov as the new Director of Clinical and Laboratory Residencies and Fellowships. Dr. Shchelochkov is an author and a co-author on more than 60 peer-reviewed publications and book chapters.



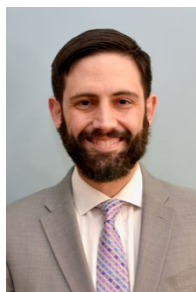
Jeffrey Siegel is the director of the Office of Drug Evaluation Sciences (ODES) in the Office of New Drugs (OND), CDER, FDA. ODES oversees Clinical Outcome Assessments, Biomarker Qualification, Research and Bioinformatics in OND. Dr Siegel has over 20 years of experience in research, regulatory, and clinical drug development. Jeff received his B.A. from Columbia University and M.D. from Yale University. He trained in internal medicine at University Hospitals of Cleveland. Then he did a fellowship in Immunology and Signal Transduction at NIH. He served at FDA from 1996-2010 as a medical officer and then Medical Team Leader. In 2010, he left FDA for industry and worked at Genentech/Roche as global lead for Rheumatology and Rare Diseases and subsequently at Gilead Sciences as Translational Medicine lead in Clinical Research/Inflammation before rejoining FDA in February, 2021.



Peter Stein is the Director of CDER'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, Dr. 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. Dr. 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.



Norman Stockbridge is Director of the Division of Cardiology and Nephrology in FDA/CDER.



David Strauss is a physician-scientist, internationally recognized researcher, and medical product regulator with over 11 years of experience at FDA spanning the Center for Drug Evaluation and Research (CDER) and Center for Devices and Radiological Health (CDRH). He currently serves as Director of FDA/CDER's Division of Applied Regulatory Science, which seeks to move new science into the FDA review process and address emergent regulatory and public health questions. The Division also conducts regulatory consults/reviews for challenging premarket or postmarket issues that cannot be addressed by the primary review divisions. He previously served as Senior Advisor for Translational & Experimental Medicine in CDER and Medical Officer in CDRH, conducting many premarket medical device reviews. Dr. Strauss received a B.A. in chemistry and M.D. from Duke University, a Ph.D. in clinical physiology from Lund University, Sweden, and additional post-doctoral training at Johns Hopkins University. He has published over 150 peer reviewed articles with >5,900 citations. He was the senior author on 3 original research JAMA articles in the past two years, including the most-read JAMA article of 2019. Additional activities include serving as Associate Editor of Clinical Pharmacology & Therapeutics, Executive Committee member of the Foundation for the National Institutes of Health Biomarker's Consortium, and Rapporteur (lead) of the International Council for Harmonisation (ICH) Guidelines for nonclinical (ICH S7B) and clinical (ICH E14) assessment of cardiac safety for drugs.



Aliza Thompson is Deputy Director of the Division of Cardiology and Nephrology, Center for Drug Evaluation and Research at the U.S. Food and Drug Administration (FDA). The Division of Cardiology and Nephrology regulates and reviews Investigational New Drug applications and marketing applications for drug and biologic products for the treatment of cardiovascular and kidney diseases. Dr. Thompson joined the FDA in 2007. Prior to her current position, Dr. Thompson served as a clinical team leader for products being developed to treat kidney diseases. Dr. Thompson received her medical degree from Johns Hopkins Medical School and completed her Internal Medicine and Nephrology training at Columbia University/New York-Presbyterian Hospital. She holds a Master of Science in Biostatistics/Patient Oriented Research Track from Columbia University Mailman School of Public Health.



Charles P. Venditti is a Senior Investigator in the National Human Genome Research Institute and the Director of the Organic Acid Research Section at the National Institutes of Health in Bethesda, MD. As a clinical biochemical geneticist and laboratorian, he has developed a translational research program to study the natural history and clinical phenotype(s) of the hereditary methylmalonic acidemias (MMA), cobalamin metabolic disorders, and propionic acidemia (PA). His research group is developing gene, cell and small molecule therapies to treat MMA and PA. Dr Venditti has received a number of national and international awards, including a Presidential Early Career Award for Scientists and Engineers (PECASE), the US Government's highest honor for early-career scientists and is an elected member of the American Society of Clinical Investigation (ASCI). He has authored and co-authored more than 130 peer reviewed research articles, reviews, and textbook chapters and is a named inventor on 21 US patents.



John A. Wagner is Chief Medical Officer at Koneksa Health. With more than 20 years of drug development experience, Dr. Wagner has led more than 150 first-in-human studies and has been instrumental bringing numerous blockbuster programs to market. Prior to joining Koneksa, John was CMO of Cygnal Therapeutics and held senior leadership roles at Takeda and Merck as well as Foresite Capital. In addition, John is Editor-in-Chief of Clinical and Translational Science and an executive committee member of the Foundation for the National Institutes of Health Biomarkers Consortium.



Steve Williams is currently responsible for Clinical R&D, Medical Affairs and Regulatory and Quality. He joined SomaLogic in 2009 as Chief Medical Officer responsible for the clinical application of the SomaScan® Platform and also had roles in launching the Life Sciences commercial business, assay development and bioinformatics. Prior to SomaLogic, Dr. Williams co-founded the pharma consultancy Decisionability, LLC in 2007 and authored the book "Decisionability." From 1989-2007, he worked at Pfizer, Inc., initially in Experimental Medicine and later as Vice President and Worldwide Head of Clinical Technology. From 2003-2007, he was on the National Advisory Council for Biomedical Imaging and Bioengineering at the National Institutes of Health. He helped to launch the Alzheimer's Disease Neuroimaging (ADNI) study and to form the FDA-FNIH-PhRMA biomarker consortium. He led or co-led the PhRMA position papers on "proof of concept," surrogate endpoints and evidentiary standards for biomarkers and diagnostics.

Dr. Williams has degrees in physiology, medicine and surgery, and a doctorate in medicine and physiology from Charing Cross and Westminster Medical School (now a part of Imperial College, London). He also obtained training in diagnostic imaging at the University of Newcastle Upon Tyne.



Lynne Yao is the Director, Division of Pediatric and Maternal Health in the Office of New Drugs, Center for Drug Evaluation and Research. Dr. Yao received a B.S. degree in Biology from Yale University, and an M.D. degree from the George Washington University School of Medicine. She is board certified in both Pediatrics and Pediatric Nephrology. Prior to joining FDA, Dr. Yao was the Director of Dialysis and Associate Pediatric Residency Program Director at the Inova Fairfax Hospital for Children in Fairfax, VA. She has been with the FDA since 2008. The Division of Pediatric and Maternal Health oversees quality initiatives which promote and necessitate the study

of drug and biological products in the pediatric population; and improve collection of data to support the safe use of drugs and biological products in pregnant and lactating individuals. She collaborates with numerous stakeholders both inside and outside of FDA to advance development of safe and effective therapies for children, and pregnant and lactating women.



Henrik Zetterberg is a Professor of Neurochemistry at the University of Gothenburg, Sweden, and University College London, UK, and a Clinical Chemist at Sahlgrenska University Hospital in Gothenburg, Sweden. He is Head of the Department of Psychiatry and Neurochemistry at the University of Gothenburg, leads the UK DRI Fluid Biomarker Laboratory at UCL and is a Key Member of the Hong Kong Center for Neurodegenerative Diseases. His main research focus and clinical interest are fluid biomarkers for brain diseases, neurodegenerative disease in particular. He has published more than 1700 scientific articles and received many awards.

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