

# Advancing Use of Patient Preference Information as Scientific Evidence in Medical Product Evaluation

## SPEAKER BIOGRAPHIES

### Welcome Session: Patient Input and Regulatory Science

**Carol Linden**, PhD, Director, Office of Regulatory Science and Innovation (ORSI), Office of the Chief Scientist, Office of the Commissioner, FDA

Dr. Linden is the Director, Office of Regulatory Science and Innovation at U.S. Food and Drug Administration. She oversees a broad array of both intramural and extramural programs focused on bringing understanding of the latest in scientific and technological advances to the process of regulating products that support the health of the American public. Prior to assuming this position, Dr. Linden was the Principal Deputy Director of the Office of the Biomedical Advanced Research and Development Authority (BARDA) in the Office of the Assistant Secretary for Preparedness and Response, Department of Health and Human Services. Her duties included oversight of advanced development and acquisition programs for Project BioShield medical countermeasures for CBRN threats as well as pandemic influenza vaccines, drugs, diagnostics and infrastructure. In 2009, Dr. Linden co-chaired with the Department of Defense the Working Group on Strengthening the Biosecurity of the United States, which was mandated by an Executive Order, and produced a report with recommendations submitted to the White House. Dr. Linden obtained her bachelor's degree in biology from Bryn Mawr College, and a Ph.D. from the University of California Los Angeles in molecular biology. She conducted postdoctoral research at the California Institute of Technology and University of Maryland prior to joining the research staff at the U.S. Army Medical Research Institute of Infectious Diseases, where she subsequently served as the Chief, Research Plans and Programs.

**RADM Denise Hinton**, Acting Chief Scientist, Office of the Chief Scientist, Office of the Commissioner, FDA

RADM Denise Hinton is FDA's acting chief scientist. In this capacity, she is responsible for leading and coordinating FDA's cross-cutting scientific and public health efforts. The Office of the Chief Scientist works closely with FDA's product centers, providing strategic leadership in the areas of regulatory science, health informatics, scientific professional development, scientific integrity, and emergency preparedness and response. RADM Hinton previously served as Deputy Director of the Office of Medical Policy (OMP) in FDA's Center for Drug Evaluation and Research (CDER), where she concurrently served as Acting OMP Director from 2014 to 2016. There, she led the development, coordination, and implementation of medical policy programs and strategic initiatives, including the efficient integration of rapidly evolving science and new technologies into the drug development and regulatory review processes. RADM Hinton's work involved close collaboration with other CDER program areas, FDA product centers, and a broad variety of stakeholders. RADM Hinton joined FDA in 2002 in CDER's Division of Cardiovascular and Renal Products and, later, served in the center's former Division of Training and Development. Before coming to FDA, she was an officer in the U.S. Air Force. RADM Hinton earned her Bachelor of Science in Nursing from Florida State University and her Master of Science degree from Boston University.

**G. Caleb Alexander**, MD, MS, Johns Hopkins Bloomberg School of Public Health, Center for Drug Safety and Effectiveness; Program Director, Johns Hopkins University CERSI

Dr. Alexander is an associate professor of Epidemiology and Medicine at the Johns Hopkins Bloomberg School of Public Health, where he serves as founding co-director of the Center for Drug Safety and Effectiveness and principal investigator of the Johns Hopkins-FDA Center of Excellence in Regulatory Science and Innovation (JH-CERSI). He is a practicing general internist and pharmacoepidemiologist and is internationally recognized for his research examining prescription drug utilization, safety and effectiveness. The author of over 200 scientific articles and book chapters, and he has published regularly in leading scientific journals, serves on several editorial and advisory boards and is a frequent speaker on pharmaceutical utilization and policy. In addition to expertise conducting survey-based investigations, Dr. Alexander also has extensive experience with the analysis of secondary data sources including administrative and pharmacy claims and large national surveys. Dr. Alexander received his B.A. cum laude from the University of Pennsylvania, an MD from Case Western Reserve University, and an MS from the University of Chicago.

### Session 1: Fundamental Concepts and Regulatory Context of PPI to Support Medical Product Development and Evaluation

**Anindita Saha**, Director, External Expertise and Partnerships, Office of the Center Director, CDRH/FDA

Anindita (Annie) Saha is the Director of External Expertise and Partnerships (EEP) in the FDA's Center for Devices and Radiological Health (CDRH). Ms. Saha leads CDRH's Patient Preference Initiative to incorporate patient perspectives on benefits and risk in regulatory decision-making. She is also helps coordinate CDRH's efforts to Partner with Patients to incorporate patient engagement and the science of patient input in device design, assessment, and review. EEP also develops and manages CDRH's external collaborations and partnerships including the Network of Experts program, public-private partnerships including the Medical Device Innovation Consortium (MDIC), fellowship programs including the Medical Device Fellowship and AIMBE Scholars programs, and technology transfer and collaboration efforts for the Center. EEP directs and coordinates CDRH's Regulatory Science and Critical

Path programs to facilitate research to promote the development and assessment of high quality, safe, and effective medical devices. Ms. Saha began her FDA career as a researcher in the CDRH's Office of Science and Engineering Laboratories in the Division of Imaging and Applied Mathematics in the area of imaging display technologies before moving to EEP. Ms. Saha has a Bachelor of Science in Bioengineering and Minor in History from the University of Pittsburgh.

**Million A. Tegenge**, RPh, PhD, Pharmacologist, Analytics and Benefit-Risk Assessment Team, Office of Biostatistics & Epidemiology, CBER/FDA

Million A. Tegenge is a pharmacologist at the U.S FDA. His regulatory review and research focuses on quantitative pharmacology/toxicology modeling, personalized medicine, pharmaceutical outcomes, patient preferences and benefits-risks evaluation of medical products. Previously, Dr. Tegenge was a neurology postdoctoral fellow at Johns Hopkins University, and received his doctoral degree from Germany in systems neuroscience.

**Bennett Levitan**, MD, PhD, Senior Director, Benefit-Risk / Epidemiology, Global R&D Epidemiology, Janssen Research & Development

Bennett Levitan, MD-PhD is Senior Director, Benefit-risk Assessment, Department of Epidemiology at Janssen R&D, Pharmaceutical Companies of Johnson & Johnson. He introduced state of the art patient-focused benefit-risk assessment J&J and has led numerous teams in benefit-risk assessments for regulatory submissions and health authority advisory meetings. He has co-led cross-disciplinary teams to implement processes to support growing regulatory requirements for patient-focused benefit-risk assessment both during development and post-approval. Bennett has published widely on both theoretical and pragmatic aspects in benefit-risk and patient preference studies and is a frequent speaker on these topics in national and international conferences. He co-led development of the PhRMA Benefit Risk Action Team (BRAT) Framework for drug benefit-risk assessment and the Medical Device Innovation Consortium (MDIC) Patient Centered Benefit-Risk Framework. Bennett serves on several committees that inform policy on benefit-risk methods including the IMI PREFER project on patient preference studies, the ISPE Benefit-Risk Assessment, Communication and Evaluation (BRACE) team, the CTTI Patient Groups & Clinical Trials work stream, the PCORI Advisory Panel on Patient Engagement and the PhRMA Patient-Focused Drug Development Work Group. Bennett received his B.Sc. (Electrical Engineering) from Columbia University in New York and his M.D.-Ph.D. (Bioengineering) from the University of Pennsylvania and was a postdoctoral fellow at the Santa Fe Institute.

**John F P Bridges**, PhD, Associate Professor, Johns Hopkins CERSI

John F P Bridges PhD is an international leader in advancing and applying methods to study the priorities and preferences of patients and other stakeholders in medicine. He is known for partnering with several patient groups to understand the benefits and risk of treatments and for his efforts to include the patients' perspective in regulatory science. He has authored of over 180 publications and is a frequent speaker on patient engagement, patient preferences, and patient-centered benefit-risk analysis. In 2008 John founded The Patient – Patient Centered Outcomes Research that is now in the top-ten most cited health policy journals. Within the International Society of Pharmacoeconomics and Outcomes Research (ISPOR) he founded the Conjoint Analysis Working Group and the Conjoint Analysis Task Force that produced several reports on good research practices for stated-preference methods. In 2006 he received ISPOR's Bernie O'Brien New Investigator Award and in 2011 received an ISPOR Distinguished Service Award. John is currently an associate professor at Johns Hopkins University and core faculty within the Center for Health Services and Outcomes Research (CHSOR), the Center for Drug Safety & Effectiveness (CDSE), and the Center for Excellence in Regulatory Science and Innovation (CERSI).

**K. Kimberly McCleary**, Acting Executive Director & Managing Director, FasterCures, A Center of the Milken Institute

Kim McCleary is Managing Director at *FasterCures*, a center of the Milken Institute determined to remove barriers to medical progress. As a member of the senior management team, Kim helps define, scope and initiate new programmatic activities and strengthen existing programs for the benefit of diverse stakeholders across the biomedical research and healthcare ecosystem. Kim leads the Patients Count: Science of Patient Input program through which *FasterCures* aims to improve health by expanding opportunities for patients' perspectives to shape the processes by which new therapies are discovered, developed and delivered. This includes advancing patient-focused medical product development, expanding patient input into regulatory decision-making and fostering shared definitions of value to improve healthcare quality, efficiency, and effectiveness while rewarding innovation in medical products and services. She works closely with leaders of The Research Acceleration and Innovation Network (TRAIN) representing mission-driven results-oriented venture philanthropy organizations to define and scale best practices and to improve the performance of the nonprofit sector to advance therapy development and patient access. Kim has also been involved in *FasterCures*' response to recent policy opportunities created by the Food and Drug Administration Safety and Innovation Act, the 21st Century Cures Act, and negotiations between industry and the FDA over programs supported by user fees for drugs and medical devices. Kim is a member of DIA's Advisory Committee for North America and the steering committee for the Medical Device Innovation Consortium's Patient-Centered Benefit Risk project. Kim is a graduate of the University of North Carolina at Chapel Hill.

## Session 2: Scientific Fundamentals of PPI Studies

**Martin Ho**, MS, Associate Director for Quantitative Innovation, Office of Surveillance and Biometrics, CDRH/FDA

**Juan Marcos Gonzalez**, PhD, Assistant Professor, Duke University, Department of Population Health Sciences, Duke Clinical Research Institute

Dr. Gonzalez is an expert in the design of stated-preference survey instruments and the use of advanced statistical tools to analyze stated-preference data. His research has focused on two main areas: 1) transparency in benefit-risk evaluations of medical interventions, and 2) elicitation of health preferences from multiple stakeholders to support shared decision making. Dr. Gonzalez Co-led the first FDA-sponsored preference study. The study was highlighted in FDA's recent precedent-setting guidance for

submitting patient-preference evidence to inform regulatory benefit-risk evaluations of new medical devices. More recently, Dr. Gonzalez collaborated with the Medical Devices Innovation Consortium (MDIC) to prepare the first catalog of preference-elicitation methods suitable for benefit-risk assessments of medical devices. The catalog was part of the Patient-Centered Benefit-Risk Assessment Framework developed by MDIC. As a core group member of the International Society for Pharmacoeconomics and Outcomes Research (ISPOR) Conjoint Analysis Task Force, Dr. Gonzalez helped draft good-practice recommendations for statistical analysis, interpretation, and reporting of health preference data. Dr. Gonzalez is also a founding member of the International Academy of Health Preference Research, and part of the leadership of ISPOR's Special Interest Group in Stated Preferences.

**Laura Lee Johnson, PhD, Director (Acting) Division of Biometrics III, Office of Biostatistics, Office of Translational Sciences, CDER/FDA**

Laura Lee Johnson, Ph.D. is an Acting Division Director and the Patient Focused Drug Development liaison for the Office of Biostatistics in CDER at FDA. She specializes in design, logistics including data collection and transmission, implementation, and analysis of research studies ranging from person reported outcome (PRO) measure qualification to safety and randomized studies of all sizes. She works across CDER and other parts of FDA on patient focused drug development initiatives. Prior to working at the FDA she spent over a decade at the U.S. National Institutes of Health working on and overseeing clinical research and research support programs including the CTSA, PROMIS, and the NIH Collaboratory.

**Leslie Wilson, PhD, Professor, Health Policy and Economics, Departments of Medicine and Pharmacy, University of California San Francisco**

Leslie Wilson is a Professor of Clinical Pharmacy and Medicine at UCSF for over 20 years and is the co-director of the Health Services and Policy Research Pathway and director of the Program for Pharmaceutical Outcome and Policy Studies at UCSF. Leslie specializes in health economics and health policy, including utility and discrete choice measurement of health preferences for drugs devices, and medical interventions. She collaborates with research scientists at FDA's Center for Devices and Radiological health (CDRH), and with support by the UCSF-Stanford Center of Excellence in Regulatory Sciences and Innovation (CERSI) developed and is testing these patient preference instruments for prosthetics. With further funding from the Burroughs Wellcome Fund she is testing the validity of different discrete choice measures for prosthetics. As a CERSI member, Leslie is also collaborating with the CDRH in workshops at UCSF and at the FDA, to increase the education of the regulatory and research community around patient preference. She is active in patient preference international organizations and a founding member of International Association of Health Preference Research (IAHPR) and is the faculty director of the ISPOR student organization at UCSF. She also has expertise in economics and outcomes studies, including comparative effectiveness, cost-effectiveness, and cost benefit analyses. I teach decision analysis at UCSF, and have expertise in decision modeling, pricing models and utility measurement. She works with California Workman's Compensation to advise on pricing models for pharmaceuticals. She also was the economist for the Center for Medicare/Medicaid Services (CMS) Innovation award to examine cost models for new systems for dementia care delivery.

**Fadia T. Shaya, PhD, MPH, Professor and Vice-Chair for Academic Affairs PHSR; Associate Director, Center on Drugs and Public Policy; University of Maryland School of Pharmacy**

Fadia T. Shaya PhD, MPH, is a tenured Professor of Pharmacoeconomics and Pharmacoepidemiology in the Department of Pharmaceutical Health Services Research at the University of Maryland School of Pharmacy, and a member of the Maryland-FDA Center for Excellence in Regulatory Science (CERSI). She is also the Executive Director of the Behavioral Health Research and Policy Program and Associate Director of the Center on Drugs and Public Policy. Prior to that, she worked at the Health Planning Commission in Paris, France. Dr. Shaya has built research capacity to support stakeholder engagement, including patients and providers, in drugs and medical devices value assessment. Her work spans all stages of drug development, evaluation and policy, from pre-clinical trials to post-marketing surveillance. She has experience developing comparative effectiveness research, clinical, economic, policy, decision analytic and budget impact models. Dr. Shaya serves on the Editorial Advisory Boards and is a referee for peer-reviewed journals such as Circulation, The Lancet, The Lancet Diabetes, The Lancet Infectious Disease, Health Affairs and Archives of Internal Medicine. She has reviewed over 500 papers and published over 220 articles. She regularly presents at national and international scientific and policy meetings, with over 200 presentations and posters to date. Dr. Shaya obtained her PhD from the Johns Hopkins Bloomberg School of Public Health, her doctoral health economics degree (DESS) from the Sorbonne University Paris-IX Dauphine in France and her Masters in Public Health (MPH) and BSc in Pharmaceutical Sciences from the American University of Beirut.

**Erica S. Spatz, MD, MHS, Assistant Professor, Cardiovascular Medicine, Center for Outcomes Research and Evaluation, Yale University School of Medicine**

Dr. Spatz is a general cardiologist and health services researcher at the Center for Outcomes Research and Evaluation (CORE), and Assistant Professor in the Section of Cardiovascular Medicine. She is an alumna of the Robert Wood Johnson Clinical Scholars Program at Yale. Dr. Spatz' research focuses on patient and health system outcomes for cardiovascular disease, and the use of shared decision making to achieve high-value care. She recently completed a Career Development Award in Patient Centered Outcomes Research from the Agency for Healthcare Research and Quality (AHRQ) to study the implementation of shared decision making. As part of this work, she developed a national measure of informed consent quality for the Centers for Medicare and Medicaid Services (CMS). She is currently funded through the National Institute of Minority Health and Health Disparities (NIMHD) and a Women's Health Research at Yale grant to study precision-based approaches to the diagnosis of cardiovascular disease, including the identification of distinct phenotypes of hypertension and of acute myocardial infarction, and their association with outcomes.

**Brett Hauber**, PhD, Senior Economist, RTI Health Solutions; Affiliate Associate Professor, University of Washington  
Brett Hauber, PhD, is Senior Economist at RTI-HS and Affiliate Associate Professor in the Department of Pharmacy at the University of Washington. He has over 20 years of experience in applied economics. Although much of his work involved discrete-choice experiments, he has extensive experience in multiple preference elicitation methods. Most of his work has involved eliciting of benefit-risk preferences and applying these results to endpoint development, assessments of unmet need, and benefit-risk assessments. His work has been included in multiple regulatory submissions. Dr. Hauber regularly teaches courses on stated preference methods. He was a member of to the Patient-Centered Benefit-Risk Steering Committee of the Medical Device Innovation Consortium (MDIC) and was principal investigator for developing the MDIC Catalog of Methods. He is a member of the scientific advisory board for the IMI-PREFER project and an advisor to a number initiatives led by industry and patient-advocacy organizations to incorporate patient preferences in regulatory and reimbursement decision making. He was chair of the ISPOR Conjoint Analysis –Statistical Analysis task force and a member of the ISPOR task force that developed the Checklist for Good Research Practices in Conjoint Analysis. His research has been published in numerous health outcomes research and medical journals.

**Becky Noel**, DrPH, MSPH, Global Benefit-Risk Leader, Global Patient Safety, Eli Lilly and Company  
Rebecca (Becky) Noel is the Global Benefit-Risk Leader at Eli Lilly and Company, where she and her team are responsible for providing benefit-risk assessment support across the Lilly portfolio. Since 2005, Becky has been extensively involved in developing and promoting systematic methods for benefit-risk assessment, both internally at Lilly and externally via the PhRMA Benefit-Risk Action Team (BRAT), the PhRMA Benefit-Risk Global Convergence issues team, the Center for Innovation in Regulatory Science (CIRS) Benefit-Risk Task Force, and two Innovative Medicines Initiative projects (PROTECT and PREFER) devoted to benefit-risk assessment and the development and use of patient preference information in benefit-risk decision making. Most recently, Becky recently served as the PhRMA Deputy for the ICH Expert Working Group responsible for the update of benefit-risk guidance in the Clinical Overview. She has contributed to multiple panels, workshops, and professional societies including FDA, IOM, DIA, ISPOR, and ISPE. Along with other Lilly colleagues, Becky also edited and contributed to the book, *Benefit-Risk Assessment in Pharmaceutical Research and Development*.

### Session 3: Discussion on In Depth Case Studies

**Million A. Tegenge**, RPh, PhD, Pharmacologist, Analytics and Benefit-Risk Assessment Team, Office of Biostatistics & Epidemiology, CBER/FDA

**Michelle Campbell**, PhD, Reviewer and Scientific Coordinator, Clinical Outcome Assessment Staff, Office of New Drugs, CDER/FDA

Dr. Michelle Campbell is a reviewer on the Clinical Outcome Assessments (COA) Staff and Scientific Coordinator of the COA Qualification Program in the Office of New Drugs (OND), Center for Drug Evaluation and Research (CDER), U.S. Food and Drug Administration (FDA). COA Staff advises OND review divisions and other FDA centers by providing consultation and advice on clinical outcome assessment development, validation, and interpretation of clinical benefit endpoints in clinical trials to support drug development, labeling, and promotion. Additionally, the COA Staff leads and manages CDER's Clinical Outcome Assessment qualification program and engages with internal and external stakeholders to advance good scientific clinical outcome measurement standards and policy development. Her prior research experience includes the use of both qualitative and quantitative methods to develop instruments, program evaluation and the application of various study designs including clinical trials. Dr. Campbell earned her BA in Biology from the College of Notre Dame, her MS in Health Science (concentration in Community Health Education) from Towson University and her PhD in Pharmaceutical Health Services Research from the University of Maryland School of Pharmacy.

**Nancy Goodman**, MPP, JD, Founder and Executive Director, Kids V Cancer

**Gregory Reaman**, MD, Associate Director (Acting) for Pediatric Oncology, Oncology Center of Excellence, Office of the Commissioner, FDA

Gregory H. Reaman, M.D. joined the Center for Drug Evaluation and Research, Office of New Drugs, U.S. Food and Drug Administration as the Associate Director of the Office of Oncology Drug Products in 2011. He is the Founding and Immediate Past Chair of the **Children's Oncology Group (COG)** having served in this capacity from 2000 through 2010. The COG is comprised of over 200 member institutions, dedicated to clinical, translational, and epidemiology research in childhood cancer. Dr. Reaman is a Professor of Pediatrics at The George Washington University School of Medicine and Health Sciences and a member of the Division of Hematology-Oncology at the Children's National Medical Center in Washington, D.C., which he directed for more than 17 years and Executive Director Emeritus of the Center for Cancer and Blood Disorders and was a member of the Board of Directors of the Children's Research Institute. Dr. Reaman serves or has served on the Editorial Boards of [Leukemia](#), [Journal of Clinical Oncology](#), [Journal of Pediatric Hematology/Oncology](#), [Pediatric Blood and Cancer](#), [The Oncologist](#), [Cancer](#), and [Physicians Data Query \(PDQ\)](#), National Cancer Institute as well as [www.PLWC.org](http://www.PLWC.org) (People Living with Cancer, now Cancer.net). Additionally, he is a member of the NCI's Pediatric Central IRB, a member of the Data Safety Monitoring Board of the National Cancer Institute's Clinical Oncology Program, and a member of the NCI's Investigational Drug Steering Committee. His research interests are in the biology and treatment of childhood acute leukemia and new drug development for pediatric cancers. He is the author of more than 300 peer - reviewed manuscripts and 16 book chapters.

**Deborah A. Marshall**, PhD, MHSA, Professor and Canada Research Chair, Health Systems and Services Research, Cumming School of Medicine, University of Calgary

Deborah Marshall holds a Canada Research Chair, Health Services and Systems Research as a Professor at the University of Calgary and Arthur J.E. Child Chair of Rheumatology Outcomes Research in the McCaig Institute of Bone and Joint Health. She

has experience in technology assessment agencies, academia and pharmaceutical and diagnostics industry research settings in Canada, the United States, and Europe. Her health technology assessment and health services research program focuses on patient preferences and patient engagement research, cost-effectiveness analysis, and simulation modeling of health services delivery to improve the effectiveness and efficiency of health care services in the context of precision medicine. She is a founding co-investigator of the innovative Patient and Community Engagement Research (PaCER) Program at the University of Calgary which trains patients to design and conduct health research, using adapted methods of qualitative inquiry. Dr. Marshall is an active member of the International Society for Pharmacoeconomics and Outcomes Research (ISPOR) as the Past President of the Board of Directors and various Task Forces, and past member of the Board of Directors for Health Technology Assessment International (HTAi).

**Paul G. Kluetz, MD**, Associate Director of Patient Outcomes (Acting), Oncology Center of Excellence, FDA

**Tamar Krishnamurti, PhD**, Assistant Professor, University of Pittsburgh School of Medicine

Tamar Krishnamurti is an Assistant Professor of Medicine at the University of Pittsburgh and holds a Ph.D. in Behavioral Decision Research from Carnegie Mellon University. Dr. Krishnamurti conducts research both domestically and internationally, applying basic knowledge to the design of communications and behavioral interventions that assist people in making more informed choices for themselves. Some of her more recent work includes the creation and evaluation of a smart mobile health intervention to providing personalized risk information to both patients and their healthcare providers during pregnancy and creating more effective methods of eliciting patient preferences for informed consent information. She publishes in a variety of peer-reviewed journals, including *PNAS*, *JAMA Internal Medicine*, *Social Science and Medicine*, *Health Psychology*, and *the Journal of Economic Behavior and Organization*.

**Elisabeth (Liz) Piauult-Louis, PharmD**, Associate Director, Patient Centered Outcomes Research Oncology, Genentech, a member of the Roche Group

Elisabeth (Liz) Piauult-Louis is an Associate Director in the Patient Centered Outcomes Research for Oncology group at Genentech, a member of the Roche Group. She has over 15 years of experience in clinical and health outcomes research working in a global healthcare value consultancy, FDA, and C-Path. She joined Genentech in 2012 where she oversees COA strategies for the Lung, CNS, Skin and GU oncology franchises and dissemination of data for regulatory approval and patients' access. Her expertise includes qualitative research for concepts identification and item development, statistical knowledge for documentation of psychometric performance of an instrument in addition to developing, analyzing and interpreting data from COA endpoints. She is passionate about capturing patients' experience with the disease and treatment to improve treatment decision-making. She is part of initiatives to advance the field of measurement through standardization of implementation and interpretation of COA endpoints in oncology trials including novel endpoints such as pain, function, treatment convenience or tolerability. Liz is PharmD with a Regulatory Affaires master from France.

**Telba Irony, PhD**, Deputy Director, Office of Biostatistics and Epidemiology, CBER/FDA

Telba Irony is Deputy Director of the Office of Biostatistics and Epidemiology at CBER, FDA. She joined FDA to implement the use of Bayesian statistics in medical device clinical trials and led the Decision Analysis initiative at CDRH, involving Bayesian statistics, benefit-risk determinations and patient preference studies. Telba received the 2014 FDA Excellence in Analytical Science Award for spearheading innovative regulatory science studies culminating in the release of novel guidance documents, supporting complex policy decision making and changing the submission review paradigm. Dr. Irony has a PhD from Berkeley, is a fellow of the American Statistical Association, and an elected member of the International Statistical Institute.

**Heather Benz, PhD**, Staff Fellow, Office of the Center Director, CDRH/FDA

Dr. Heather Benz is a Medical Device Fellow in the Center for Devices and Radiological Health (CDRH) at the U.S. Food and Drug Administration. She conducts research on patient preferences in the Office of the Center Director in support of the Center strategic priority "Partnering with Patients," with a focus on the application of patient preference information to neurological device review. She also collaborates with researchers in the Center's Office of Science and Engineering Laboratories on outcome measures for advanced upper limb prostheses. Dr. Benz received a B.S. in Biomedical Engineering from Case Western Reserve University, Cleveland, Ohio and a Ph.D. in Biomedical Engineering from Johns Hopkins University School of Medicine, Baltimore, Maryland.

**Catherine Kopil, PhD**, Director, Research Partnerships, The Michael J. Fox Foundation

Katie Kopil joined The Michael J. Fox Foundation (MJFF) in 2013. As Director of Research Partnerships, Katie leads strategic stakeholder engagement – including patients and industry – throughout the R&D continuum building ongoing relationships with key partners in therapy development and care delivery for Parkinson's disease. She has developed and oversees of programs critical to increasing patient engagement in Parkinson's research including the Fox Trial Finder matching tool and the Fox Insight study of PROs. In her previous capacity as a staff scientist in Research Programs at MJFF, Katie drove efforts to identify and develop biomarkers of Parkinson's disease and collaborated on landmark clinical cohort studies including the Parkinson's Progression Markers Initiative (PPMI). Katie earned an undergraduate degree in Psychology from Princeton University and a PhD in Neuroscience from the University of Pennsylvania. Prior to joining the Foundation, Katie completed her postdoctoral research training in Bioengineering in a brain injury laboratory at the University of Pennsylvania. Katie brings her broad knowledge-base in cellular/molecular neuroscience and translational medicine in addition to clinical trials experience to the Foundation to help bring new treatments to people with Parkinson's.

**Ellen M. Janssen, PhD**, Assistant Scientist, Department of Health Policy and Management, Johns Hopkins Bloomberg School of Public Health

Ellen M. Janssen, PhD, is a health economist focused on valuing healthcare interventions and outcomes. She has worked with quantitative and qualitative methods to value the cost of behavioral interventions and to measure the preferences and priorities of patients across a wide spectrum of healthcare questions including treatment preferences, communication strategies, and risk prioritization. Ellen is passionate about engaging patients as research partners in all levels of healthcare decision-making. As such she has worked with a variety of patient and community groups, as well as regulatory experts at FDA in her research. Her current work examines good research practices for the development, implementation, analysis, and evaluation of stated-preference methods to ensure that patient preference studies are transparent and meet high quality standards.

**Ira Shoulson, MD**, Professor of Neurology, Pharmacology and Human Science; Director, Program for Regulatory Science & Medicine (PRSM): Principal Investigator, Georgetown University CERSI

Ira Shoulson, MD is Professor of Neurology, Pharmacology and Human Science and Director of the Program for Regulatory Science and Medicine (PRSM) at Georgetown University, Washington, DC (<http://regulatoryscience.georgetown.edu>). He received his MD degree and postdoctoral training in medicine and neurology at the University of Rochester and in experimental therapeutics at the NIH. Dr. Shoulson founded the Parkinson Study Group ([www.parkinson-study-group.org](http://www.parkinson-study-group.org)) in 1985 and the Huntington Study Group ([www.huntington-study-group.org](http://www.huntington-study-group.org)) in 1994 -- international academic consortia devoted to research and development of treatments for Parkinson disease and Huntington disease. He was a key investigator in the US-Venezuela Collaborative Huntington Disease Project, which identified the gene responsible for this fatal hereditary disorder. He played an instrumental role in the development of 10 new drugs for neurological disorders, including seven for Parkinson disease, two for Huntington disease, and one for attention deficit disorder. In 2016, he was recipient of the Michael J. Fox Foundation Pritzker Prize, in recognition of his leadership in research and education for Parkinson disease. Dr. Shoulson is currently principal investigator of the FDA-Georgetown University Collaborating Center of Excellence in Regulatory Science and Innovation (CERSI - FD004319), and an elected member of the National Academy of Medicine.

**Kerry Jo Lee, MD**, Medical Officer, Guidance and Policy Team, Immediate Office, Office of New Drugs, CDER/FDA

Kerry Jo Lee is a graduate of Princeton University and of the New York University School of Medicine with an honors degree conferred in microbiology. She completed her pediatric residency 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. During this time, she completed research involving the microbiome and viral pathogens at the Center for Infection and Immunity at Columbia University. Dr. Lee has also worked for several years in bioethics with the National Bioethics Advisory Commission on reports surrounding ethical and policy issues in both international and domestic clinical trials and interned at the World Health Organization. Upon joining the FDA, Dr. Lee was a reviewer in the Division of Gastroenterology and Inborn Errors Products where she contributed to papers published on pediatric trial design in inflammatory bowel disease and pediatric drug development. Currently she works as a medical officer on the Guidance and Policy Team in the Office of New Drugs within the Center for Drug Evaluation and Research at the U.S. Food and Drug Administration as team lead for benefit-risk analysis and support for reviewers.

**Janel Hanmer, MD, PhD**, Assistant Professor of Medicine, University of Pittsburgh; Medical Director, UPMC Patient Reported Outcomes Center

Dr. Hanmer is a general internist and the medical director of the patient reported outcomes center at the University of Pittsburgh Medical Center. She has a PhD in Population Health Sciences with a focus in health-related quality of life measurement and the measurement of preferences for health.

**Kara L. Haas, MD, MPH, FACS, RAC**, Global Regulatory Affairs Policy and Intelligence, Medical Device Evidence and Outcomes, Johnson & Johnson

Kara Haas, M.D., M.P.H., FACS is the Global Lead for Regulatory Affairs and Policy for Medical Device Outcomes and Evidence at Johnson & Johnson. Her role is focused on issues and topics related to regulatory policy and practices addressing clinical and real world evidence and outcomes including patient centered initiatives and diversity in trial populations.

**Kathryn M. O'Callaghan**, CDRH Assistant Director for Strategic Programs, Office of the Center Director, CDRH/FDA

**Andrea Ferris**, President and CEO, LUNGevity Foundation

Andrea is President and CEO of LUNGevity Foundation. She became involved with lung cancer advocacy following her mother's death from the disease in 2008. After receiving a diagnosis of stage IV lung cancer in 2006, Andrea's mother underwent numerous treatments and clinical trials at several major academic institutions to no avail. Together with her father, Andrea was her mother's primary caregiver during this time. Determined to drive more money into lung cancer research, Andrea left the successful software company that she helped launch, to found Protect Your Lungs, an organization focused 100% on funding early detection research. In 2010, Andrea merged Protect Your Lungs with LUNGevity, a Chicago based organization, to form the nation's leading lung cancer focused non-profit. Andrea's strong business background combined with her connections to the worlds of research and advocacy have enabled her to build the preeminent patient advocacy organization in the lung cancer space. LUNGevity funds translational research into both early detection and more effective treatments of lung cancer as well as a highly coveted Career Development Awards program. LUNGevity also fills unmet needs for people diagnosed with lung cancer by providing education, support and survivorship programs. Recognizing the need to build awareness and understanding about lung cancer, LUNGevity has built the largest grassroots network of events and advocates across the country.

## Day 2 Welcome

**Theresa M. Mullin**, PhD, Director, Office of Strategic Programs, CDER/FDA

## Session 4: CDRH Preference Sensitive Areas Discussion: Diseases and Conditions where patient preference studies could be useful

**Heather Benz**, PhD, Staff Fellow, Office of the Center Director, CDRH/FDA

**Vishal Bhatnagar**, MD, Medical Officer, Office of Hematology Oncology Products, CDER/FDA

Vishal Bhatnagar, MD, is a medical officer in the Office of Hematology and Oncology Products at the U.S. Food and Drug Administration. His work focuses on the evaluation of investigational new drugs and marketing applications for drugs for the treatment of malignant hematologic disorders. His regulatory interests include patient preference and incorporation of patient experience in oncology trials. He also serves as a scientific liaison for multiple myeloma, which involves engagement with the multiple myeloma community. Dr. Bhatnagar received his BA in Political Science and his medical degree at the George Washington University. He completed his internal medicine residency and hematology/oncology fellowship at the University of Maryland.

**Million A. Tegenge**, RPh, PhD, Pharmacologist, Analytics and Benefit-Risk Assessment Team, Office of Biostatistics & Epidemiology, CDER/FDA

**Liana Fraenkel**, MD, MPH, Professor of Medicine, Yale University School of Medicine

Dr. Fraenkel is Professor of Medicine at Yale University School of Medicine, Chief of Rheumatology of VA Connecticut Healthcare System and Associate Program Director, Section of Rheumatology, Yale School of Medicine. As a health services researcher, she conducts research to improve our understanding both physician and patient decision-making and to develop methods to improve the quality of decision making for patients faced with complex decisions involving multiple options. In 2009, Dr. Fraenkel received the Henry Kunkel Young Investigator Award from the ACR given annually to a rheumatologist  $\leq 45$  years of age for outstanding research in this field. Dr. Fraenkel has area of expertise is in the field of medical decision-making and risk communication. She has conducted numerous studies incorporating qualitative and quantitative methodologies that evaluate patient attitudes towards drug toxicity, physician and patient treatment preferences, and the contextual influences on risk perceptions.

**Stephanie Christopher**, MA, Program Director, Science of Patient Input, Medical Device Innovation Consortium

Stephanie Christopher, MA, has a background in health communication, public health project management and communication education and training. Stephanie joined MDIC in 2013 and manages MDIC's patient centered benefit-risk assessment, patient engagement and quality initiatives. Prior to joining MDIC, Stephanie worked for seven years with an academic public health team working on interventions to improve the quality of communication between physicians and parents of newborns with abnormal newborn screening results. In 2012-13, Stephanie went on leave from her academic position to do a special assignment for the Food and Drug Administration Center for Devices and Radiological Health (CDRH), updating and training staff on a new risk communication process. Stephanie has also served as an adjunct instructor at Marquette University, teaching introductory communication courses. Stephanie earned her Bachelor of Arts in Communication-Print Journalism from Pacific Lutheran University in Tacoma, Wash. and Master of Arts in Science, Health, and Environmental Communication from Marquette University in Milwaukee, Wis. Stephanie is also a Certified Clinical Research Coordinator (CCRC) through the Association of Clinical Research Professionals.

**Melissa West**, Project Director, Kidney Health Initiative

Melissa West is currently the Project Director for the Kidney Health Initiative (KHI). KHI is a public private partnership between the American Society of Nephrology (ASN), FDA, and over 80 companies and organization focused on kidney disease. Ms. West has served the kidney community for over 15 years--first as the Director of the American Society of Nephrology's Kidney Week program (which is the largest scientific meeting in nephrology), then as Project Manager for Medical Education at Abbott Laboratories, and finally as a consultant for Reata Pharmaceuticals and AMAG Pharmaceuticals. She returned to ASN in 2012 to launch the Kidney Health Initiative.

**Frank Hurst**, MD, Medical Officer, Renal Devices Branch, Division of Reproductive, Gastro-Renal, and Urological Devices, Office of Device Evaluation, CDRH/FDA

Dr. Hurst currently serves as a Medical Officer in the Renal Devices Branch at FDA's Center for Devices and Radiological Health. Prior to joining FDA, he was a nephrologist in the US Army, serving at the Walter Reed Army Medical Center in Washington, DC. He is Board Certified in Internal Medicine and Nephrology.

## Session 5: Capacity Building and Sustainability

**Michelle Tarver**, MD, PhD, Ophthalmologist/Epidemiologist; Division of Ophthalmic and Ear, Nose, and Throat Devices; Office of Device Evaluation, CDRH/FDA

Michelle Tarver is a medical officer at the Food and Drug Administration in the Center for Devices and Radiological Health. She attended Spelman College in Atlanta, GA where she received a B.S. in biochemistry. She completed the MD/PhD program at The Johns Hopkins University Bloomberg School of Public Health and the Johns Hopkins School of Medicine. She is board certified in ophthalmology and fellowship trained in ocular immunology. Following her residency and fellowship training at the Wilmer Eye Institute, she remained there on the Ophthalmology faculty as an Assistant Professor. She joined the Food and Drug Administration in 2009 where she has been actively involved in research on ophthalmic devices and efforts aimed at incorporating the patient's voice in the evaluation of medical devices. She led the development of a patient-reported outcome measure for LASIK surgery and glaucoma and continues to be involved with developing these measures for other ophthalmic conditions. She has also led

the development of a patient preference study for glaucoma. She has received numerous awards for her research efforts from both the FDA and the American Academy of Ophthalmology.

**Cynthia Grossman, PhD, Associate Director, Science of Patient Input, *FasterCures***

Dr. Cynthia (Cyndi) Grossman is associate director, Science of Patient Input at *FasterCures*, a center of the Milken Institute leading efforts to improve health by expanding opportunities for patients' perspectives to shape the processes by which new therapies are discovered, developed and delivered. Prior to joining *FasterCures*, Dr. Grossman was chief of the HIV Care Engagement and Secondary Prevention Program at the National Institute of Mental Health (NIMH). At NIH, she worked on collaborative efforts to define the social and behavioral scientific agenda for the development and clinical testing of microbicides as HIV prevention and HIV cure related research. She has spent nearly two decades encouraging research to identify and address the unmet patient needs related to mental health, stigma, and other social determinants of health. Dr. Grossman graduated Phi Beta Kappa from Earlham College with a BA in psychology and biology and earned her PhD in clinical psychology from the University of Vermont. Dr. Grossman has been the recipient of a National Science Foundation Incentives for Excellence Scholarship, an NIH Ruth L. Kirschstein National Research Services Award, and a Postdoctoral Fellowship in Pediatric Psychology at the Warren Alpert Medical School of Brown University.

**Ebony Dashiell-Aje, PhD, Reviewer, Clinical Outcome Assessments Staff, Office of New Drugs, CDER/FDA**

Dr. Dashiell-Aje is a reviewer with the Clinical Outcome Assessments (COA) Staff in the Center for Drug Evaluation and Research (CDER) at the FDA. She is responsible for reviewing qualitative and quantitative research in various therapeutic areas to provide advice and consultation in CDER. During the past 15 years, Dr. Dashiell-Aje has notably contributed her social science research expertise in academic, consulting and regulatory environments to arrive at evidence-based solutions.

**Matthew Reaney, CPsychol, CSci, MSc; Global Head of Clinical Outcomes, Sanofi**

Matt is a Chartered and Practitioner Health Psychologist, a Chartered Scientist, a Fellow of both the Royal Societies of Medicine and Public Health, and an Associate Fellow of the British Psychological Society. Matt is the Global Head of Clinical Outcomes at Sanofi, focusing on understanding and measuring patient-relevant outcomes and experiences in a scientifically sound way. This includes both outcome evaluation for clinical drug development and the support of patients in routine clinical practice. Matt is particularly interested in understanding and embracing patient heterogeneity in defining outcome measures such as benefit-risk and patient perception. In addition to his work at Sanofi, Matt retains academic positions in the UK.

**Shelby D. Reed, PhD, Professor in Population Health Sciences and Medicine, Preference Evaluation Research (PrefER), Group Duke Clinical Research Institute**

Shelby Reed, PhD, RPh is Professor in Population Health Sciences and Medicine at Duke University and Director of the Preference Evaluation Research (PrefER) Group at the Duke Clinical Research Institute. Dr. Reed has 20 years of experience leading multidisciplinary health outcomes research studies. Dr. Reed has extensive expertise in designing and conducting trial-based and model-based cost-effectiveness analyses of diagnostics, drugs and patient-centered interventions. In evaluating health policy issues, she has developed computer models to evaluate the economic impact of trends in clinical trial design, changes in reimbursement policies, a new financing scheme to spur drug development for ultra-rare conditions, and the societal value of alternative approaches to identifying drug safety problems. Over the last several years, her research has increasingly focused on stated-preference studies to evaluate benefit-risk tradeoffs, patient-centered value, and their application in comparative effectiveness research and clinical decision making. Dr. Reed earned pharmacy and doctoral degrees from the University of Maryland and completed her training in the Pharmaceutical Outcomes Research and Policy Program at the University of Washington. She serves on editorial advisory boards for *Value in Health* and *Health Services Research*. She is currently serving as President of the International Society for Pharmacoeconomics and Outcomes Research (ISPOR).

**C. Daniel Mullins, PhD, Professor, Pharmaceutical Health Services Research Department (PHSR), University of Maryland School of Pharmacy**

C. Daniel Mullins, PhD is a Professor and Chair of the Pharmaceutical Health Services Research Department at the University of Maryland School of Pharmacy. He directs the University of Maryland PATient-centered Involvement in Evaluating effectiveNess of TreatmentS (PATIENTS) Program, which is an infrastructure to support patient-centered outcomes research and related training activities. He has received funding as a Principal Investigator from the NIH/NIA, NIH/NHLBI, AHRQ, the Patient-Centered Outcomes Research Institute (PCORI) and various patient advocacy and industry organizations. He has served as a Regular Member of AHRQ and NCI Study Sections and has chaired PCORI Study Sections. Professor Mullins is co-Editor-in-Chief for *Value in Health* and is author/co-author of more than 200 peer-reviewed articles. In 2013, he was the recipient of the Dr. Daniel D. Savage Memorial Science Award, the Association of Black Cardiologists' most prestigious annual award. Also in 2013, he was awarded a University System of Maryland Wilson H. Elkins Professorship. He was named Researcher of the Year in 2014 and received the Martin Luther King Faculty Diversity Award in 2017 for the University of Maryland Baltimore campus and the ISPOR Marilyn Dix Smith Leadership Award.

**Joseph S. Ross, MD, MHS, Associate Professor of Medicine (General Medicine) and Public Health (Health Policy and Management), Yale University**

**R. Scott Braithwaite, MD, MS, FACP, Chief, Division of Comparative Effectiveness and Decision Sciences; Professor of Population Health and Medicine, Department of Population Health, New York University School of Medicine**

R. Scott Braithwaite, MD, MS, FACP is a Tenured Professor of Population Health and Medicine, Director of the Division of Comparative Effectiveness and Decision Sciences at New York University School of Medicine. He was the founder of the



Comparative Effectiveness Research Training Program. After studying physics at M.I.T., Dr. Braithwaite earned his MD from the State University of New York at Stony Brook, trained in internal medicine at the University of Washington, and received an MS in Clinical Research from the University of Pittsburgh. Dr. Braithwaite also completed a fellowship in Clinical Decision Making at Tufts University and is a recipient of a prestigious Robert Wood Johnson Foundation Faculty Scholar award. As Director of the Division of Comparative Effectiveness and Decision Sciences, he is dedicated to advancing a program of rigorous, policy-relevant research to optimize quality and value in healthcare, incorporating methods of decision science, comparative effectiveness and cost effectiveness. He is an accomplished investigator in the field of decision science, quality and cost-effectiveness with an outstanding record of funding from the NIH and other extramural sources. In addition to focusing on the improvement of domestic care for chronic illness, he also continues his important international work on HIV treatment strategies in developing countries.

**Frank F. Weichold**, MD, PhD, Director, Critical Path and Regulatory Science Initiatives, Office of Regulatory Science and Innovation, Office of the Chief Scientist, Office of the Commissioner, FDA

Dr. Weichold is the director for Critical Path and Regulatory Science Initiatives in the office of the Chief Scientist and the Office of the Commissioner for the US-Food and Drug Administration. The expertise he brings to the regulatory agency builds on his ability to advance, coordinate, and integrate scientific resources for FDA by addressing mission critical scientific regulatory challenges in a global environment. The FDA Centers of Excellence in Regulatory Science and Innovation (CERSI) network is being built under Dr. Weichold's leadership in collaboration with academic institutions to leverage scientific expertise, resources and capacity toward FDA's mission. He represents FDA at the Maryland Life Science Advisory Board and at the NIH National Center for Advancing Translational Sciences. Dr. Weichold's experience includes execution of strategic and operational initiatives across the sciences' value chain. He has led the development of international collaborations and public private partnerships for discovery and early medical product development, implemented global operating and development models, and executed large-scale business model transformations. He has accumulated more than a decade of industrial research and medical product development experience while leading teams in Clinical Pharmacology, DMPK, as a Director at MedImmune LLC, and AstraZeneca. Prior, he directed research and clinical development of vaccines at the Aeras Foundation (founded by The Bill and Melinda Gates Foundation). As a tenured Professor in the University of Maryland system, he developed and managed independent research programs and trained graduate students. His medical practice and clinical experience includes Infectious Diseases and Immunology/Rheumatology.

**Telba Irony**, PhD, Deputy Director, Office of Biostatistics and Epidemiology, CBER/FDA