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Enhancing the Development and Use of Patient-Reported Outcomes in Drug Development

Embassy Row Hotel • Washington, DC
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Biographies

Ethan Basch, MD is a practicing oncologist and Director of the Cancer Outcomes Research Program at the University of North Carolina in Chapel Hill. His research focuses on patient-reported outcomes, clinical informatics, and comparative effectiveness. Studies by his group have determined that patient self-reporting of adverse events can improve data accuracy and comprehensiveness compared to clinician reporting. Building on this work, he leads the National Cancer Institute's PRO-CTCAE initiative to develop a standardized patient-centered approach to safety reporting in clinical trials. He serves as a member of the Methodology Committee of the Patient-Centered Outcomes Research Institute (PCORI) for which he co-chairs the Patient-Centeredness Workgroup. He is a member of the Board of Scientific Advisors of the National Cancer Institute, chairs the Health Outcomes Committee of the Alliance for Clinical Trials in Oncology, and is a member of the Board of Directors of the International Society for Quality of Life Research. The overall goal of Dr. Basch's work is to improve our understanding of, and the quality of, patients' experiences with illness and care.

Cynthia Bens is the Vice President of Public Policy at the non-profit Alliance for Aging Research in Washington, D.C. In this capacity, Ms. Bens is responsible for guiding the organization's federal policy work, representing the Alliance in multiple national coalitions, and directing all aspects of the Accelerate Cure/Treatments for Alzheimer's Disease (ACT-AD) and Aging in Motion (AIM) coalitions. Ms. Bens sits on the Alliance for a Stronger FDA Board of Directors and is a Founding Executive Committee Member of Friends of the National Institute on Aging (FoNIA). She has served on the Steering Committee of the Partnership to Improve Patient Care (PIPC) since 2008 and is the sole patient adviser to the FDA Subcommittee of BIOCOM, the largest regional life science association in the world. For the past 12 years Ms. Bens has worked to inform federal policymakers and educate the public on a variety of issues. For more than half of that time her efforts have centered on the formulation of policies to expedite the development of interventions to treat and prevent many debilitating age-related disease; to remove access barriers to needed treatments and therapies; and to improve the coordination and quality of care seniors receive. Prior to joining the Alliance in 2006, Ms. Bens was a senior manager of government affairs with the Loeffler Group. As part of its federal government affairs practice, she represented diverse client interests before the U.S. Congress and the administration. Her core areas of focus included appropriations, budget, health care, education, telecommunications, and international trade. Through various other positions on Capitol Hill and in the private sector, she has acquired extensive experience researching and analyzing federal legislation and regulations. Ms. Bens holds a Bachelors of Arts degree from New York University with concentrations in Political Science and Women's Studies

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Marc Boutin, Esq. is the executive vice president and chief operating officer of the National Health Council, an organization that brings together all segments of the health care community to provide a united voice for the more than 133 million people with chronic diseases and disabilities and their family caregivers. In addition to overseeing financial management and operations at the National Health Council, Boutin builds consensus among member patient advocacy organizations enabling them to speak with one voice on systemic health research and health care policy initiatives. This united effort results in legislation and regulations that address the collective needs of patients and their family caregivers. In addition, he provides guidance to patient organizations on various association issues, including corporate structure, government relations, fundraising, and outreach. Boutin has been actively involved in health advocacy, policy, and both federal and state legislation throughout his career. He is a member the International Alliance of Patients' Organizations Governing Board, Community Health Charities Board of Directors, PCORI Advisory Panel on Patient Engagement, Sanofi Partners in Patient Health Global Council, and the North America Advisory Board to the Drug Information Association.

Stephen Joel Coons, PhD is Executive Director of the Patient-Reported Outcome (PRO) Consortium at the Critical Path Institute (C-Path). C-Path, an independent, non-profit organization, established the PRO Consortium in cooperation with the U.S. Food and Drug Administration and the pharmaceutical industry in 2008. Stephen joined C-Path after a 23-year career in academia. His last academic role was professor in the College of Pharmacy and the College of Public Health at the University of Arizona. In addition, he served as co-director of the Arizona Cancer Center's Behavioral Measurements Shared Service. After receiving a BS in pharmacy from the University of Connecticut, Stephen earned an MS in pharmacy, an MEd in higher education, and a PhD in pharmacy (administrative and behavioral sciences) at the University of Arizona. His post-doctoral training in health outcomes research was completed at the University of California, San Diego (UCSD). Previous academic appointments have been in the colleges of pharmacy, medicine, and allied health professions at the University of Kentucky and at the UCSD School of Medicine. Stephen is a fellow in the American Association of Pharmaceutical Scientists and an emeritus professor at the University of Arizona. For over two decades, the primary focus of his research has been the measurement of patient-reported outcomes.

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Gregory Daniel, PhD, MPH, RPh, is a fellow in Economic Studies and managing director for evidence development and innovation in the Engelberg Center for Health Care Reform at the Brookings Institution. In this position, Dr. Daniel oversees and provides strategic direction regarding the Center's evidence development and biomedical innovation portfolio, including medical product safety surveillance, regulatory science and U.S. Food and Drug Administration (FDA) policy issues, comparative effectiveness research, and other biomedical innovation policies. Dr. Daniel was previously Vice President, Government and Academic Research at HealthCore (subsidiary of WellPoint, Inc.) where he led a division responsible for providing research services in the areas of pharmacoepidemiology; drug, vaccine, and biologic safety evaluations; comparative effectiveness research; and health economics and outcomes research. His research has utilized electronic health insurance claims data integrated with clinical data including laboratory results, electronic hospital data, paper-based and electronic medical record data, and registries. Dr. Daniel is a registered pharmacist and holds a PhD in pharmaceutical economics, policy, and outcomes research with a minor in epidemiology from the University of Arizona. He also holds an MPH specializing in biostatistics, a master's in pharmaceutical administration, and a BS in pharmacy, all from The Ohio State University.

Amylou Dueck, PhD, is joined Mayo Clinic in Rochester, Minnesota, as a faculty statistician in 2005 after receiving a doctoral degree in statistics from Arizona State University. In May 2007, she transferred to the Mayo Clinic in Scottsdale, Arizona, where she is the current head of the Section of Biostatistics in the Division of Health Sciences Research. Dr. Dueck's primary role at Mayo Clinic is designing and analyzing cancer clinical trials and associated lab-based and patient-reported outcomes-based correlative studies. Dr. Dueck is also the co-vice chair of the Health Outcomes Committee of the Alliance for Clinical Trials in Oncology. Her primary research interests include development/validation of patient-reported outcomes (specifically, the MPN-SAF and PRO-CTCAE) and analysis/interpretation issues of patient-reported outcomes.

Pat Furlong is the Founding President and CEO of Parent Project Muscular Dystrophy (PPMD), the largest nonprofit organization in the United States solely focused on Duchenne muscular dystrophy (Duchenne). Their mission is to end Duchenne. They accelerate research, raise their voices in Washington, demand optimal care for all young men, and educate the global community. Duchenne is the most common fatal, genetic childhood disorder, which affects approximately 1 out of every 3,500 boys each year worldwide. It currently has no cure. When doctors diagnosed her two sons, Christopher and Patrick, with Duchenne in 1984, Pat didn't accept "there's no hope and little help" as an answer. Pat immersed herself in Duchenne, working to understand the pathology of the disorder, the extent of research investment and the mechanisms for optimal care. Her sons lost their battle with Duchenne in their teenage years, but she continues to fight—in their honor and for all families affected by Duchenne. In 1994, Pat, together with other parents of young men with Duchenne, founded PPMD to change the course of Duchenne and, ultimately, to find a cure. Today, Pat continues to lead the organization and is considered one of the foremost authorities on Duchenne in the world.

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Deb Gipson, MD, MS is an Associate Professor of Pediatrics and Director of the Honest Broker Office of the University of Michigan School of Medicine. She is the principal investigator for patient reported outcomes (PRO) research in nephrotic syndrome, chronic kidney disease and obesity. In partnership with the NephCure Foundation patient advocacy group, she developed and co-led a 2014 workshop on the development of PROs for use in nephrotic syndrome which included patients, FDA, PCORI, NIH and academia. In PRO, clinical trial, decision support, translational, educational and observational research studies, patients are engaged in the design, governance and dissemination activities within the research portfolio under Dr. Gipson's leadership.

Ari Gnanasakthy, MSc, MBA is Head of Patient Reported Outcomes at RTI Health Solutions. Prior to his recent appointment at RTI-HS Ari was an Executive Director at Novartis Pharmaceuticals, and headed the Patient Reported Outcomes Center of Excellence. He has been in the pharmaceutical industry for almost 25 years. Within Novartis he has been in various functions including Biostatistics, Health Economics, Pricing, and Outcomes Research. After receiving his bachelor degree in Mathematics, Statistics and Computing from University of Greenwich, UK, Ari was at Rothamsted Experimental Station (UK) where he was responsible for the statistical analysis of a survey data of agricultural soil in England and Wales, and then at the Milk Marketing Board (UK) where he was part of a team that was responsible for modeling lactation curves of dairy cows. Ari also has a Master's degree in Applied Statistics from Sheffield Hallam University (UK) and an MBA from The University of Kingston (UK). Ari's extensive experience in the field of statistics and outcome research has resulted in numerous abstracts and almost 40 publications. Throughout his career Ari has developed and validated over a dozen patient reported outcomes instruments and currently serves as a reviewer for many professional journals including Value in Health.

Chad Gwaltney, PhD focuses on the development of innovative methods to measure patient-centered outcomes in clinical trials. He has published numerous articles and book chapters addressing how the patient's perspective can be examined to better understand product efficacy and safety in areas such as addictive behaviors, diabetes, cardiovascular health, dermatology, and dental medicine. He has served on multiple international task forces and US National Institutes of Health review committees. He has co-authored best practice guidelines on the content validity of new patient-reported outcome measures and migrating clinical outcome assessments from paper to electronic platforms. His academic and industry research includes the design of electronic platforms for the collection of information in real-time in the patient's natural environment. He received his Ph.D. in Clinical-Health Psychology from the University of Pittsburgh and is currently on the research faculty in the Department of Behavioral and Social Sciences at Brown University.

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Katarina Halling, MSc is Patient Reported Outcomes group Director at AstraZeneca and heads up the global PRO Centre of Excellence. In that role, she and the PRO team collaborates with the broader organization to develop and implement relevant PRO/COA strategies and plans in support of global development programs across AZ/MedImmune within respiratory&inflammation, oncology, cardiovascular&metabolism and nesuroscience. Katarina has more than 20 years of experience of incorporating the patient voice in drug development, both within AstraZeneca and for 3 years as a consultant. As a consultant, Katarina was the scientific and regulatory lead for PRO and ePRO in Europe with PRO Consulting. During her career, Katarina has developed several PRO instruments, to address efficacy, tolerability and impact of treatments in several diseases as well as diagnostic PRO tools and communication tools to improve communication between patient and physician. She has extensive ePRO experience and has interacted on PRO measurement strategies with FDA, EMA, PMDA and sFDA. Katarina is passionate about basing PRO strategies on solid science and cross pharma collaborations to increase the efficiency and visibility of PROs in drug development. Katarina is behavior scientist with MSc in Psychology.

Nancy Kline Leidy, PhD has over 25 years of experience in clinical and health outcomes research, specializing in instrument selection, development and testing, and the design and analysis of clinical trials involving patient-centered outcomes. Dr. Leidy has presented her work internationally and has over 115 scientific publications. She served as director and principal investigator of the EXACT-PRO Initiative, the first PRO instrument development consortia. This multi-sponsor initiative brought together experts in measurement, clinical research, and regulatory issues to develop the EXACT and EXACT-RS for measuring outcomes in COPD trials. The EXACT was the first PRO instrument to undergo qualification review by the FDA and the first qualified (January 10, 2014). The E-RS is under review. Dr. Leidy is an active member of ISPOR and ATS, among others. She served on ISPOR task forces addressing regulatory requirements for the use of PRO information in labeling and good research practices to assure content validity of PRO instruments (*Value in Health*, 2003, 2009, 2011). She is also an advisor to the European Innovative Medicines Initiative (IMI) PROactive project developing physical activity outcome measures for COPD trials. Dr. Leidy holds degrees from Michigan State University (Bachelors), University of Washington (Masters), and The University of Michigan (PhD).

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Mark McClellan, MD, PhD, is a senior fellow and director of the Initiative on Value and Innovation in Health Care at the Brookings Institution. Within Brookings, his work focuses on promoting quality and value in patient centered health care. A doctor and economist by training, he also has a highly distinguished record in public service and in academic research. Dr. McClellan is a former administrator of the Centers for Medicare & Medicaid Services (CMS) and former commissioner of the U.S. Food and Drug Administration (FDA), where he developed and implemented major reforms in health policy. These include the Medicare prescription drug benefit, the FDA's Critical Path Initiative, and public-private initiatives to develop better information on the quality and cost of care. Dr. McClellan chairs the FDA's Reagan-Udall Foundation, is co-chair of the Quality Alliance Steering Committee, sits on the National Quality Forum's Board of Directors, is a member of the Institute of Medicine, and is a research associate at the National Bureau of Economic Research. He previously served as a member of the President's Council of Economic Advisers and senior director for health care policy at the White House, and was an associate professor of economics and medicine at Stanford University.

Theresa M. Mullin, PhD, as Director of the CDER Office of Strategic Programs Dr. Mullin leads CDER strategic planning and directs the CDER international program, business informatics, drug data standards, program and strategic analysis. This includes leading FDA development of a drug benefit-risk assessment framework, Patient Focused Drug-Development initiative, and a new effort to establish the first formal pharmaceutical quality surveillance capability in CDER. In addition, Dr. Mullin heads the FDA delegation to ICH leading the US discussion of the future of ICH. She served as the FDA lead negotiator for the 2012 reauthorization of the Prescription Drug User Fee Act (PDUFA) providing an estimated $700 million in annual fee revenues. She also served as lead negotiator for new user fees for biosimilar biological products authorized under the Biologics Price Competition and Innovation Act of 2009. Prior to joining CDER in September 2007, Dr. Mullin was Assistant Commissioner for Planning in the FDA Office of Commissioner where she led FDA user fee negotiations with the pharmaceutical industry for both the 2007 and the 2002 reauthorizations of PDUFA. Since joining FDA, Dr. Mullin has received a number of awards including the Senior Executive Service Presidential Rank Award for Distinguished Service in 2011 and Presidential Rank Award for Meritorious Service in 2006, as well as the FDA Commissioner's Award of Excellence. Prior to work at FDA, Dr. Mullin was a Senior Manager with The Lewin Group, specializing in health care consulting, and Principal Scientist at Decision Science Consortium, specializing in decision research and analysis. Dr. Mullin received her B.A. *magna cum laude* in Economics from Boston College, and Ph.D. in Public Policy Analysis from Carnegie-Mellon University.

Elektra Papadopoulos, MD, MPH, currently serves as the lead medical officer for CDER's Study Endpoint and Labeling Development (SEALD) Study Endpoints team. She first joined SEALD in 2007 as a reviewer on the Study Endpoints team, which provides advice on the development and implementation of clinical outcome assessments for use in trials to support medical product approval and labeling claims. Since joining SEALD, Elektra has participated in the development of guidance for the regulatory qualification of clinical outcome assessments. She is a board certified dermatologist with experience in the review and design of clinical trials. She has served as an FDA Medical Officer since 2001, first in the Center for Biologics Evaluation and Research (CBER) and subsequently also in the Division of Dermatology and Dental Products in CDER. Beginning when she was still at CBER, Elektra was involved with patient reported outcome (PRO) endpoint review issues and on the development of the FDA PRO guidance document as a member of the FDA PRO Working Group.

Donald Patrick, PhD has worked on outcomes for over 45 years. He is Professor of Health Services at the University of Washington with appointments in Epidemiology, Pharmacy, Rehabilitation Medicine, and Sociology. He is a Full Member of the Fred Hutchinson Cancer Research Center. He is internationally recognized as a leading developer and user of multiple generic and disease-specific measures. He directs the Seattle Quality of Life Group (www.seaqolgroup.org) and works on projects funded by the National Institutes of Health, AHRQ, and Industry. He is active in research on health outcomes, health disparities youth QoL, and people with disabilities. He is a member of the Institute of Medicine As a Special Government Employee, he contributed to the FDA Guidance on Patient-Reported Outcome Measures for Use in Medical Product Development and Labeling Claims. He is actively involved in Task Forces and other work for both the International Society for Quality of Life Research, for which he was inaugural president, and the nternational Society for Pharmacoeconomics and Outcomes Research, who conferred the Donabedian lifetime achievement award in 2012. He is author of numerous articles and monographs, a classic book written with Pennifer Erickson entitled Health Status and Health Policy and a book with Richard Deyo entitled Hope or Hype: The Obession with Medical Advance and the High Cost of False Promises.

Debra Silberg, MD, PhD is currently a Senior Director at Shire leading the GI Clinical Development programs. Prior to joining Shire, Dr. Silberg was a Global Medical Science Director at AstraZeneca. As part of her work in industry, she has been involved in the development of several clinical outcome assessments (patient and observer reported outcome instruments). In addition, she is currently a member of the C-Path working group for functional dyspepsia. Dr. Silberg has served as the industry representative to the GI Division Advisory Committee at the FDA. She earned her Bachelor of Science degree at the University of Michigan, her Ph.D. in Immunology from Wayne State University School of Medicine, and her M.D. from Albert Einstein College of Medicine. She completed her Internal Medicine Residency and Gastroenterology Fellowship at the University of Pennsylvania Health System. She then became a faculty member in the Department of Medicine, Division of Gastroenterology at the University of Pennsylvania where she treated patients and was the principal investigator of an NIH funded molecular biology laboratory. Dr. Silberg is an active member in the American Gastroenterological Association, participating on the AGA research awards review panel.

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Tara Symonds, PhD is the PRO Lead at Pfizer providing advice on PRO strategy across the portfolio. She has worked at Pfizer in various roles within Outcomes Research since 1998, prior to this she worked in academia teaching undergraduate and postgraduate psychology. Tara has more than 19 years of experience in the development and implementation of PROs for use in clinical research. Numerous of the questionnaires and screening tools she has worked on across a variety of therapeutic areas, can be accessed at www.pfizerpatientreportedoutcomes.com. Tara was recently elected as the co-director of the C-PATH PRO Consortium. She is co-author of the newly published book: Cappelleri et al (2014) Patient Reported Outcomes: Measurement, Implementation and Interpretation, CRC Press. Tara is a Chartered Health Psychologist and holds a Doctorate in Health Psychology from the University of Huddersfield, UK.

Ellis F. Unger, M.D. is the Director of Office of Drug Evaluation-I, Office of New Drugs, Center for Drug Evaluation and Research (CDER) at FDA. Dr. Unger obtained his medical degree from the University of Cincinnati. He completed internal medicine training at the Medical College of Virginia and a fellowship in cardiology at Johns Hopkins. From 1983 to 1997, Dr. Unger directed a translational research program in angiogenesis at NIH, where he worked to develop new approaches for the treatment of coronary artery disease and peripheral vascular disease. In 1997, Dr. Unger joined FDA's Center for Biologics Evaluation and Research, serving as a Medical Officer, Team Leader, and subsequently Branch Chief in the Office of Therapeutics Research and Review. With reorganization of CDER in 2005, Dr. Unger became Deputy Director of the Division of Cardiovascular and Renal Products. From 2007 to 2008, he served as the Acting Deputy Director of CDER's Office of Surveillance and Epidemiology. He became Deputy Director, Office of Drug Evaluation-I, in July, 2009, and became its Director in July, 2012. Dr. Unger has served on numerous working groups, including CIOMS Working Group VII, and the ICH Expert Working Groups on E2F and E2C (R2). He has authored and co-authored numerous scientific articles, and is a co-holder of two patents.

James P. Witter, MD, PhD, FACR is the Program Director of the Rheumatic Diseases Clinical Program at the National Institute of Arthritis and Musculoskeletal and Skin Diseases (NIAMS) at the National Institutes of Health (NIH), and the Chief Science Officer of the Patient-Reported Outcomes Measurement Information System (PROMIS), an initiative of the NIH Roadmap for Medical Research. He received his Ph.D. in Medical Microbiology/Immunology with a minor in Bacteriology from the University of Wisconsin Medical School, and his medical degree from the Medical College of Wisconsin. He is a Harvard-trained, board-certified rheumatologist who is a member of several ACR committees. Before joining NIH, Dr. Witter was a Medical Officer at the Food and Drug Administration (FDA), and a Staff Physician in Rheumatology at the National Naval Medical Center. Dr. Witter has received multiple awards and honors in his career, including the NIH Director's Award, and is a frequent presenter at professional rheumatology meetings. At NIAMS, Dr. Witter manages a research portfolio that supports patient-relevant clinical research and the design, development and execution of clinical trials and registries in rheumatic diseases in adults and children, such as rheumatoid arthritis, lupus, scleroderma, ankylosing spondylitis, psoriatic arthritis, gout, inflammatory myopathies, and vasculitis.