Clinical Outcome Assessments: Establishing and Interpreting Meaningful Within-Patient Change
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Biographies

Wen-Hung Chen is currently a reviewer in the Clinical Outcome Assessment Staff in the Office of New Drugs (OND), Center for Drug Evaluation and Research (CDER), FDA. Dr. Chen’s responsibilities include supporting the review divisions in issues related to the development and interpretation of study endpoints, and supporting the drug development tool qualification program. His areas of expertise include psychometric data analysis and clinical outcome assessments. He was involved with the development of the pain item bank for the NIH leaded patient reported-outcome measurement information system (PROMIS) project. He was also involved in the development of the Exacerbations of Chronic Pulmonary Disease Tool (EXACT) developed under the EXACT-PRO Initiative. Dr. Chen earned his Bachelor's degree in Science from Chengchi University in Taipei, Taiwan; his Master's degree and Doctorate in quantitative psychology, with a minor in Biostatistics, from University of North Carolina at Chapel Hill. His publications have appeared in Journal of Educational and Behavioral Statistics, British Journal of Mathematical and Statistical Psychology, Journal of Educational Measurement, Journal of Pain Symptom and Management, Pain, Quality of Life Research, Clinical Drug Investigation, European Respiratory Journal, Chest, and Health Outcomes Research in Medicine.

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. Michelle 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.
Karon Cook is a Research Professor at the Feinberg School of Medicine, Northwestern University, Chicago, IL. Her work has focused both on applications of modern psychometric approaches and on advancing methodological science. Dr. Cook has invested substantial efforts in recent years promoting the need for better methods for interpreting scores on health outcome measures. She and her colleagues at Northwestern have applied approaches developed in educational testing to the interpretation of scores from health measures. This has included applying a “Bookmarking” method to develop cut scores for symptom severity levels (e.g., no problems, moderate, mild, severe) in the context of patient reported outcomes. She has recently applied similar methods in the estimation of thresholds for meaningful change scores. Dr. Cook has been an investigator on NIH PROMIS-funded projects for the last 12 years and has served as principle investigator on multiple grants and contracts funded by National Institutes of Health, Agency for Healthcare Research and Quality, the Department of Defense, and VA Health Services and Rehabilitation Research. She is also known for her efforts in developing accessible webinars and presentations on the science of PROs.

Stephen Joel Coons 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 over 20 years 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.

Gregory Daniel is a Clinical Professor in Duke's Fuqua School of Business and Deputy Director in the Duke-Robert J. Margolis Center for Health Policy at Duke University. Dr. Daniel directs the DC-based office of the Center and leads the Center’s pharmaceutical and medical device policy portfolio which includes developing policy and data strategies for improving development and access to innovative pharmaceutical and medical device technologies. This includes post-market evidence development to support increased value, improving regulatory science and drug development tools, optimizing biomedical innovation, and supporting drug and device payment reform. Dr. Daniel is also a Senior Advisor to the Reagan-Udall Foundation for the FDA and Adjunct Associate Professor in the Division of Pharmaceutical Outcomes and Policy at the UNC Eshelman School of Pharmacy. Previously, he was Managing Director for Evidence Development & Biomedical Innovation in the Center for Health Policy and Fellow in Economic Studies at the Brookings Institution and Vice President, Government and Academic Research at HealthCore (subsidiary of Anthem, Inc). Dr. Daniel’s research expertise includes utilizing electronic health data in designing research in health outcomes and pharmacoconomics, comparative effectiveness, and drug safety and pharmacoepidemiology. Dr. Daniel received a PhD in pharmaceutical economics, policy and
outcomes form the University of Arizona, as well as an MPH, MS, and BS in Pharmacy all from The Ohio State University.

**Selena Daniels** serves as a Team Leader for the Clinical Outcome Assessments (COA) Staff in the Office of New Drugs (OND), Center for Drug Evaluation and Research (CDER), U.S. Food and Drug Administration (FDA). The COA Staff provides consultation services to OND review divisions and other centers on COA endpoint development, validation, interpretation and representation in labeling and advertising for individual drug development programs, as well as to instrument developers under CDER’s COA Drug Development Tool Qualification Program. Selena leads a team of expert analysts who evaluate a variety of complex study endpoint issues related to the evaluation of clinical benefit in clinical trials to support labeling and marketing claims. Before joining the COA Staff, Selena was a Senior Manager in the Global Health Outcomes Strategy and Research department at Allergan, Inc. where she was responsible for developing and executing health economic and outcome research (HEOR) strategies to support Allergan’s clinical development programs in Dermatology, Aesthetics, and Plastics. She has a decade of experience in HEOR research including developing and implementing innovative COA strategies and endpoints for clinical trials. Selena earned her Bachelor of Science degree in Sports Medicine from Pepperdine University and her Doctor of Pharmacy degree from Loma Linda University. After pharmacy school, she completed a post-graduate year 1 (PGY1) pharmacy residency at the Medical Center of Central Georgia and HEOR Fellowship (sponsored by Allergan, Inc.) at the University of Arizona where she earned her Master of Science degree in Pharmaceutical Sciences (emphasis in Pharmacoeconomics, Policy, and Outcomes).

**Dana Britt DiBenedetti** is Executive Director of Patient-Centered Outcomes Assessment at RTI-HS, is a licensed clinical health psychologist with extensive experience in the assessment of adults and children in numerous therapeutic areas. Her broad clinical and assessment experience includes expertise in adult and pediatric psychiatry, hemophilia, women's health (e.g., menopause, vaginal atrophy, endometriosis), sexual medicine (e.g., erectile dysfunction, female sexual dysfunction, arousal disorders, Peyronie’s disease), dermatology, diabetes, virology (hepatitis C), and substance abuse. Dr. DiBenedetti specializes in the development of questionnaires designed to measure symptom severity, quality of life, treatment satisfaction, and other patient- and caregiver-reported outcomes. She also has significant experience in the design and implementation of survey studies in various patient and health care professional populations. As part of the questionnaire-development process, Dr. DiBenedetti has facilitated numerous patient, caregiver, and physician focus groups, and conducted cognitive interviews with a wide variety of patient and professional populations. Dr. DiBenedetti has considerable experience in both academic and pharmaceutical settings with instrument development and validation, functional status and quality-of-life assessment. She has authored numerous manuscripts and scientific presentations on instrument development, quality of life, treatment satisfaction, and other outcomes in various patient populations, and she has served as a reviewer for several journals, including Value in Health, Clinical Therapeutics, Journal of Consulting and Clinical Psychology, Health Psychology, Annals of Behavioral Medicine, International Journal of Behavioral Medicine, Addictive Behaviors, and Medical Care.
Chris Edgar is a Senior Outcomes Research Scientist in the Patient-Centered Outcomes Research group at Roche, based in the UK. He holds PhD in psychopharmacology from Northumbria University and has worked in the pharma industry for the past 17 years. This has included positions as scientific director of a cognition assessment CRO, a consultant to pharma supporting Alzheimer’s clinical trial design and OR, a senior clinical lead for rater training and data quality, and 3 years as a clinical scientist at Roche working on Schizophrenia and Alzheimer’s drug development programs. Chris has a strong background in CNS and neuroscience drug development, with a focus on PerfO, ClinRO and ObsRO. Chris is a Co-Chair of the C-Path Cognition working group. Current research interests include defining clinically meaningful progression in neurodegenerative disease, cognitive screening for Alzheimer’s disease, application of survival analysis to neuroscience trials, and defining treatment benefit for negative symptoms associated with schizophrenia. Chris’s expertise on meaningful change estimates has been applied across the business, including Oncology. Chris’s measurement expertise is well recognized within the company and he is the OR lead for the Alzheimer’s and Parkinson’s disease programs at Roche and Genentech.

Chad Gwaltney develops 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 medical product efficacy and safety. He has served on multiple international task forces and US National Institutes of Health review committees and has co-authored best practice guidelines on the development and use of patient-reported outcomes. 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.

Laura Lee Johnson is Deputy Director of FDA CDER’s Division of Biometrics III and the Clinical Outcome Assessment liaison for CDER’s Office of Biostatistics. She provides guidance on design, logistics, implementation, and analysis of research studies ranging from person reported outcome measure qualification to safety and randomized studies of all sizes. Dr. Johnson serves on the FDA-NIH Interagency Clinical Outcome Assessments Working Group and co-directs the NIH Principles and Practice of Clinical Research course. Prior to working at the FDA she spent over a decade at the NIH working on and overseeing clinical research and research support programs. She has been involved with numerous projects developing, validating, and using clinical outcome assessments in both patient care and research. Dr. Johnson received her Ph.D. in Biostatistics from the University of Washington.

Lisa Kammerman is a Senior Statistical Science Director and Regulatory Specialist in the Oncology Therapeutic Area, Biostatistics and Information Sciences, AstraZeneca. She helps guide the design and analysis of clinical trials that use patient-reported outcomes in oncology and other therapeutic areas. She developed AstraZeneca’s PRO guidance document and also led the development of AstraZeneca’s best practices document on missing data. Prior to AstraZeneca, Dr. Kammerman was a biostatistician at the Food and Drug Administration FDA and retired after 24 years of public health service. She was a Master Reviewer in the Division of Biometrics III, Center for Drug Evaluation and Research and oversaw the Office of Biostatistics’ Clinical Outcome Assessments program. She helped develop policy for the design, analysis, and
interpretation of studies that use Clinical Outcome Assessments. In addition, she belonged to numerous committees including CDER’s Science Prioritization and Review Committee and the Pediatric Review Committee, FDA’s Institutional Review Board, and numerous guidance document working groups (e.g., labeling, non-inferiority, site selection for inspections, rare diseases). Dr. Kammerman provided statistical support in many medical areas, including anti-viral, anti-infective, special pathogen, pulmonary, reproductive, urologic, gastrointestinal, inborn errors of metabolism and dermatological medical products.

Mark McClellan is the Robert J. Margolis Professor of Business, Medicine, and Policy, and Director of the Duke-Margolis Center for Health Policy at Duke University with offices at Duke and in Washington DC. The new Center will support and conduct research, evaluation, implementation, and educational activities to improve health policy and health, through collaboration across Duke University and Health System, and through partnerships between the public and private sectors. It integrates the social, clinical, and analytical sciences to integrate technical expertise and practical capabilities to develop and apply policy solutions that improve health and the value of health care locally, nationally, and worldwide. Dr. McClellan is a doctor and an economist, and his work has addressed a wide range of strategies and policy reforms to improve health care, including such areas as payment reform to promote better outcomes and lower costs, methods for development and use of real-world evidence, and more effective drug and device innovation. Before coming to Duke, he served as a Senior Fellow in Economic Studies at the Brookings Institution, where he was Director of the Health Care Innovation and Value Initiatives and led the Richard Merkin Initiative on Payment Reform and Clinical Leadership. 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, Medicare and Medicaid payment reforms, the FDA’s Critical Path Initiative, and public-private initiatives to develop better information on the quality and cost of care. Dr. McClellan is the founding chair and a current board member of the Reagan-Udall Foundation for the FDA, is a member of the National Academy of Medicine and chairs the Academy’s Leadership Council for Value and Science-Driven Health care, co-chairs the guiding committee of the Health Care Payment Learning and Action Network, and is a research associate at the National Bureau of Economic Research. He has also 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 as Deputy Assistant Secretary for Economic Policy at the Department of the Treasury. He was previously an associate professor of economics and medicine with tenure at Stanford University, and has twice received the Kenneth Arrow Award for Outstanding Research in Health Economics.

Elektra Papadopoulos is the Associate Director for the Clinical Outcome Assessments (COA) Staff in the Office of New Drugs, Center for Drug Evaluation and Research, FDA. The COA Staff contributes to a culture that ensures the patient voice is integrated into drug development through COA endpoints that are meaningful to patients, valid, reliable and able to detect meaningful change. The Staff works collaboratively to provide consultation for COAs including patient-reported outcomes used across all stages of drug development, manages the COA Drug Development Tool Qualification Program to develop and qualify COAs for unmet public health needs, and provides education and outreach to internal and external stakeholders to advance the science of COA development and implementation in drug development. Elektra joined the Staff (formerly known as SEALD, Study Endpoints Team) in 2007 as a Study Endpoints
reviewer before becoming Acting Team Leader in 2013 and subsequently Acting Associate Director in 2015. She has participated in the development of guidance for the regulatory qualification of COAs and has overseen the development of an initiative to publish a compendium of COAs to promote collaboration, transparency, and communication between FDA and its stakeholders. She has also contributed to the writing of numerous guidance documents. Elektra is a board certified dermatologist. She obtained her medical degree at the University of Texas Medical Branch in Galveston. She completed her dermatology residency at the University of Texas Medical Center in Houston and dermatology fellowship at the Dermatology Branch of the National Cancer Institute before serving as Assistant Professor of Medicine and Dermatology at Georgetown University Medical Center in Washington, DC. In 2001, she joined FDA as a Medical Officer, first in the Center for Biologics Evaluation and Research and subsequently in the Division of Dermatology and Dental Products in CDER.

David Thissen is a professor in the Department of Psychology and the L.L. Thurstone Psychometric Laboratory at the University of North Carolina at Chapel Hill. His research interests are in the areas of psychological testing, measurement, and item response theory (IRT). His 2001 book with Howard Wainer, Test Scoring, describes both traditional and novel uses of IRT to compute scores for conventional linear tests, tests comprising mixtures of item types, and computerized adaptive tests (CATs). He is one of the authors of Computerized Adaptive Testing: A Primer. He was primary developer of the widely used computer software Multilog, and one of the authors of IRTPRO, both flexible systems for item calibration using IRT models. He is currently involved in research programs that use IRT to measure aspects of health-related quality of life, and adaptive behavior for individuals with intellectual disabilities. He also works in various roles with a number of educational assessment programs.