ENGELBERG CENTER for
Health Care Reform
at BROOKINGS

Advancing Development and Use of Patient-Reported Outcomes in Drug Development: Near-Term Opportunities

Brookings Institution • Washington, DC
Monday, October 6, 2014

Biographies

Naomi Aronson, PhD, is the Executive Director of Clinical Evaluation, Innovation, and Policy, Blue Cross and Blue Shield Association. Dr. Aronson has overseen the development of the Blue Cross Blue Shield Association Technology Evaluation Center (TEC) as a nationally recognized technology assessment program and an Evidence-based Practice Center (EPC) of the Agency for Healthcare Research and Quality (AHRQ). She has directed over 300 technology assessments and 20 evidence reports for AHRQ. Dr. Aronson is a member of the Methodology Committee of the Patient-Centered Outcomes Research Institute (PCORI). Dr. Aronson is a member of the Health Technology Assessment International Health Policy Forum, the Institute of Medicine Genomics Roundtable, and the National Business Group on Health Committee on Evidence-Based Benefit Design. Previously, she has represented the private sector on the U.S. Agency for International Development Team providing technical assistance to the Hungarian government on building evidence-based medicine capacity and also served on the Ontario Health Technology Assessment Evaluation Review Team. She was a member of the Institute of Medicine Forum on Drug Discovery Translation and Development, and a review committee co-chair for the International society for Pharmacoeconomics and Outcomes Research Annual Meeting. Prior to joining TEC, Dr. Aronson was a member of the Northwestern University faculty, specializing in the sociology of science and medicine. She also was a post-doctoral fellow in the Science, Technology and Society Program at the Massachusetts Institute of Technology and received research awards from the National Science Foundation and the American Council of Learned Societies. Dr. Aronson's academic research focused on how the organization of scientific specialties in biomedical and clinical research affects the process of scientific discovery.

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.

Alicyn Campbell is currently the Global Head of Patient Centered Outcomes Research for Oncology at Genentech, a role she has held since 2012. She has over 9 years of experience developing and implementing innovative PRO strategies and endpoints across tumor types within oncology drug development (e.g. non-small cell lung cancer, head and neck cancer, hematological malignancies, gastric cancer, glioblastoma, ovarian cancer, hepatocellular carcinoma, breast cancer [from neo-adjuvant to metastatic], renal cell carcinoma, and pancreatic cancer). Her current research includes: assessment of symptom burden in lung cancer, comparative tolerability assessment, and the development of new tools for neurocognitive assessment of brain metastases, as well as the CNS effects of treatment. Alicyn currently is co-chair of the C-Path NSCLC Working Group. Prior to joining Genentech Alicyn was a Director in Health Economics and Outcomes Research at Pfizer Oncology for 6 years, where she was responsible for Outcomes Research strategy for the development pipeline, including PRO endpoints. Additionally, Alicyn spearheaded and lead a company-wide initiative to develop integrated treatment management guidelines for common toxicities across small molecules, as well as designed a novel treatment management intervention trial using PROs as primary endpoint of efficacy. She completed her Masters of Public Health from the University of Connecticut School of Medicine with concentrations in Epidemiology and Outcomes Research. Alicyn is passionate about measuring the impact of cancer and its treatment on patients in a rigorous and reliable way to provide patients with the best evidence to make one of the most important healthcare decisions they will face in their life time: choice of treatment following a cancer diagnosis.

Charles S. Cleeland, PhD, is Chair, Department of Symptom Research at M. D. Anderson Cancer Center. He and his group are the developers of assessment measures for cancer-related pain and other symptoms, and the functional impairment caused by these symptoms, including the Brief Pain Inventory and the MD Anderson Symptom Assessment Inventory. His work in pain and symptom assessment in cancer has been recognized by the American Pain Society, with its 2003 Fordyce Award for clinical investigation, and by the American Cancer Society, with its Trish Greene Quality of Life Award for his commitment to improving the quality of life of patients. Dr. Cleeland has been principal investigator on numerous federal grants. He serves as an advisory board member or consultant to several pharmaceutical companies in the assessment of symptoms in developing new oncologic drugs. Together with Dr. Jeff Sloan, Dr. Cleeland cofounded an interdisciplinary workgroup of stakeholders in optimal symptom assessment to review the status of symptom measurement in cancer clinical trials using patient-reported outcomes. The mission of this group, ASCPRO (Assessing the Symptoms of Cancer using Patient-Reported Outcomes), is to generate evidence-based recommendations for the assessment of patient-reported cancer-related symptoms to facilitate clinical research and decision-making.

ENGELBERG CENTER for
Health Care Reform
at BROOKINGS

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.

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 biomedical innovation portfolio that develops practical policy solutions in the areas of drug, device, and biologic policy, regulatory science, and evidence generation, including post-market safety surveillance and comparative effectiveness research. Dr. Daniel is also a Senior Advisor to the Reagan-Udall Foundation for the FDA. Previously, Dr. Daniel was 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, 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, a MPH specializing in biostatistics, a MS in Pharmaceutical Administration, and a BS in Pharmacy, all from The Ohio State University.

Robert H. Dworkin, PhD, received his B.A. in 1971 from the University of Pennsylvania and his Ph.D. in 1977 from Harvard University. He is currently Professor of Anesthesiology, Neurology, Oncology, and Psychiatry, Professor of Neurology in the Center for Human Experimental Therapeutics, and Director of the Anesthesiology Clinical Research Center at the University of Rochester School of Medicine and Dentistry. Dr. Dworkin is Director of the Analgesic, Anesthetic, and Addiction Clinical Trial Translations, Innovations, Opportunities, and Networks (ACTTION) public-private partnership with the US Food and Drug Administration (FDA); a member of the US Centers for Disease Control and Prevention Zoster Working Group; and a Special Government Employee of the FDA Center for Drug Evaluation and Research. He is an Associate Editor of *Pain*, a member of the Editorial Boards of *Journal of Pain* and *Current Pain and Headache Reports,* and has previously served as a consultant to and member of the FDA Anesthetic and Life Support Drugs Advisory Committee. Dr. Dworkin received the American Pain Society's Wilbert E. Fordyce Clinical Investigator Award in 2005, the Eastern Pain Association's John J. Bonica Award in 2011, and the American Pain Society's John and Emma Bonica Public Service Award in 2014. The primary focus of Dr. Dworkin's current research involves methodologic aspects of analgesic clinical trials, especially identifying factors that might increase the assay sensitivity of a trial to detect differences between an active and a control or comparison treatment. With research funding from the FDA and industry, he and colleagues are currently examining in acute and chronic pain trials the relationships between study methodologic features and study outcomes, as well as comparing the responsiveness to treatment effects of different primary and secondary outcome measures. The overall objective of these efforts -- which are being conducted under the auspices of the ACTTION public-private partnership -- is to identify approaches to improving the efficiency and informativeness of clinical trials of pain treatments and provide the foundation for an evidence-based approach to analgesic clinical trial design.

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.

ENGELBERG CENTER for
Health Care Reform
at BROOKINGS

Glenn Kroog, MD, is a Medical Oncologist by training and a Group Director in Oncology Global Clinical Research at Bristol-Myers Squibb (BMS). Dr. Kroog currently leads the clinical development of 2 drugs primarily being studied in hematologic malignancies. Prior to joining BMS in 2010, Dr. Kroog spent 3 years as an Assistant Member at Memorial Sloan-Kettering Cancer Center (MSKCC) focusing on clinical care and clinical research for patients with kidney cancer. Prior to joining MSKCC, Dr. Kroog spent 7 years as an Assistant Professor primarily running a research laboratory at Albert Einstein College of Medicine in New York which focused on G protein-coupled receptor signal transduction. Dr. Kroog holds a BA from Yale University, an MD from Cornell University Medical College, completed an Internal Medicine residency at New York Hospital-Cornell Medical Center, and a Medical Oncology fellowship at the National Cancer Institute.

J. Jason Lundy, PhD, is the Director of the Electronic Patient-Reported Outcomes (ePRO) Consortium at the Critical Path Institute. In this role, he is responsible for the overall management of the Consortium, which includes establishing the scientific activities, as well as coordinating the electronic implementation of the instruments being developed in the Patient-Reported Outcome (PRO) Consortium. He is also the Associate Director of the PRO Consortium. In this role, he assists the Director with the overall management of the consortium, and provides scientific input/expertise to the industry working groups during all stages of the PRO instrument development process. Previously, Dr. Lundy worked as a Senior Scientist in a large contract research organization, managing the design, implementation, analysis, and reporting of patient-reported outcome studies.

Mark B. 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.

Josephine Norquist is the Patient-Reported Outcome (PRO) Specialist and chair of DeVISE* Center of Excellence (CORE – Epidemiology at Merck Sharp & Dohme, Corp.). In her role Josephine leads, develops and implements endpoint strategies for global clinical trials across therapeutic areas. She leads regulatory strategies and interactions with agencies on Clinical Outcome Assessments (COAs) to support labeling claims. She is a representative on the C-PATH PRO Consortium, chairs the ISOQOL Industry Advisory Committee (IAC) and is part of the Study Endpoints Special Interest Group within DIA. Before joining Merck, she worked at the University of Oxford, Institute of Health Sciences in Oxford, U.K., where she was a Research Officer responsible for statistical analyses, report writing, and manuscript preparation in the areas of PROs. Prior to that, she was a Research Statistician at the Center on Outcomes, Research and Education (CORE) at Evanston Northwestern Healthcare, Evanston, IL. She earned her B.S. in Statistics and Economics from the Universita' degli Studi di Palermo (Italy) and her M.S. in Statistics from Northwestern University (IL). Josephine's primary focus is developing and validating COAs, specifically to support labeling claims. She has written many peer-reviewed journal articles and has created several endpoint dossiers for submissions to regulatory agencies. *=**De**velopment, **V**alidation, **I**mplementation and **S**tandardization of clinical trial **E**ndpoints

Jean Paty, PhD, is Senior Director and Practice Lead for Endpoint Strategy within Global Market Access & Commercialization at Quintiles Consulting. Dr. Paty is an acknowledged leader in the effective strategies and practices of capturing patient perspective data for use in the clinical development and commercial success of new medical products. He has not only published extensively in the areas of Patient Reported Outcomes (PRO) and electronic PRO (ePRO), but also on the regulatory guidance for development and implementation of ePRO. He has worked closely with the international industry and regulatory agencies on ePRO best practices. His work is well-referenced in a wide variety of peer-reviewed journals and in numerous conferences and events, where he has presented his findings on the scientific, clinical, and regulatory implications of Clinical Outcome Assessment (COA) data collection in clinical trials. Dr. Paty has a B.S. in Psychology from the University of Toronto and an M.S. and Ph.D. in Psychology from the University of Pittsburgh. He can be reached at jean.paty@quintiles.com**.**

Bryce B. Reeve, PhD, Dr. Bryce Reeve is an Associate Professor within the Department of Health Policy and Management, Gillings School of Global Public Health, University of North Carolina at Chapel Hill (UNC-CH). He is also a member of the UNC Lineberger Comprehensive Cancer Center and Research Fellow with the Cecil G. Sheps Center for Health Services Research. Trained in psychometrics, his work focuses on enhancing the application of patient-reported outcomes (PROs) in clinical research and practice to improve the quality of care for pediatric and adult cancer patients. This includes the development of PRO measures using qualitative and quantitative methodologies and integration of PRO data in research and healthcare delivery to inform decision-making. Prior to his faculty position with UNC, Dr. Reeve served as a Program Director for the National Cancer Institute from 2000 to 2010. He recently completed 2 years of service as President of the International Society for Quality of Life Research (ISOQOL). Dr. Reeve is a Principal Investigator (PI) on two NIH-funded R01 grants, an NIH-funded U01 grant, and a PCORI-funded contract.

ENGELBERG CENTER for
Health Care Reform
at BROOKINGS

Arthur Stone, PhD, was trained as a clinical psychologist and is currently Professor of Psychology and Director of the USC Dornsife Center for Self-Report Science at the University of Southern California. He is also Emeritus Distinguished Professor of Psychiatry and Behavioral Science at Stony Brook University. Stone's early work was concerned with improving the measurement of life events and coping with the goal of understanding how events and coping impact our susceptibility to somatic illnesses. These studies led to an interest in psychobiology with a particular emphasis on how environmental events affect the immune system and the endocrine system. At the same time he was researching how people self-report information about their psychological and symptom states. This led to the development of various kinds of daily diaries that measured end-of-day and within-day phenomena, which ultimately yielded a set of techniques known as Ecological Momentary Assessment. More recently, Stone has been involved with the development of alternative methods for capturing the ebb and flow of daily experience for large-scale surveys, including the development of the Day Reconstruction Method. He is also been involved with the development of questionnaires for use in clinical trials, which has been supported by a consortium from the National Institutes of Health.

Dennis C. Turk, PhD, Dr. Turk is the John and Emma Bonica Professor of Anesthesiology and Pain Research and Director of the Center for Pain Research on Impact, Measurement, & Effectiveness (C-PRIME) at the University of Washington. He is Past President of the American Pain Society. Dr. Turk has received a number of awards including Recipient of the Award for Outstanding Scientific Contributions to Health Psychology from the American Psychological Association and the Wilbert E. Fordyce Clinical Investigator Award from the American Pain Society. Dr Turk is currently Editor-in-Chief of *The Clinical Journal of Pain* and Co-chair of the Initiative on Methods, Measurement, & Pain Assessment in Clinical Trials (IMMPACT) and Co-Director of the Executive Committee for the Analgesic, Anesthetic, and Addiction Clinical Trials Translations, Innovations, Opportunities, & Networks (ACTTION) initiative – a public-private partnership with the FDA. He currently was a member of the Institute of Medicine's Committee on Advancing Pain Research, Care, and Education. He has contributed over 565 publications and authored or edited 20 volumes, most recently Chronic Pain: An Integrate Biobehavioral Approach (with H. Flor), The Pain Survival Guide: How to Reclaim Your Life (with F Winter), the 3rd edition of the *Handbook of Pain Assessment* (with R. Melzack), From Acute to Chronic Back Pain: Risk Factors, Mechanisms, and Clinical Implications (with M. Hasenbring, A. Rusu), Wall & Melzack's Textbook of Pain, 6th edition (with S. McMahon, M. Koltzenburg, I Tracey), and the 3rd edition of Practical Management of Pain (with H onzon, J Rathmell, C Wu, C Argoff, & R Hurley). An international survey, published in The Pain Clinic in 2001, designated Dr. Turk as "One of the 10 "Leading Contributors to the Field of Pain".