Leveraging Randomized Clinical Trials to Generate Real-World Evidence for Regulatory Purposes

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Biographies

Nawar Bakerly is the clinical director for the Department of Respiratory Medicine (Pulmonology) and the Divisional Chief Clinical Information Officer (CCIO) at Salford Royal Foundation Trust, Greater Manchester, UK; as well as being the lead for integrated COPD services. Dr Bakerly also works as an academic at the University of Manchester, and has participated as lead or co-investigator in many clinical trials including being a co-investigator on the Salford Lung Studies for Asthma and COPD. He has an interest in Real World Evidence and pragmatic RCTs, and in using technology to improve clinical care and facilitate research. Over the last 13 years, he has been involved in developing and evaluating innovative technologies in health care, including the development of smartphone applications for primary care physicians to assist in the management of asthma and COPD, as well as contributing to the development of digital clinical pathways as part of the NHS Global Digital Exemplar (GDE) programme. His other interests include connecting digital health care systems for clinical and research use.

Greg Ball didn’t take a straight path to becoming a statistician. After graduating from Northwestern University with a bachelor’s in economics, he served in the Navy for four years and taught high school math and physics for five years, before going back to school to get a master’s in applied statistics from Purdue University. Eventually, while working as a statistician, he earned his PhD in Biostatistics from the University of Texas School of Public Health in Houston, Texas. At Merck, he was the main driver of a cross-disciplinary company-wide initiative to develop an innovative new aggregate safety assessment planning process. He has created, developed and implemented a procedure, with a collaborative process and a quantitative framework, for review of aggregate blinded clinical trial data to support safety signal detection and risk management activities. Externally, he has established the ASA Biopharm Safety Monitoring working group (with Bill Wang), co-led the Interdisciplinary Safety Evaluation workstream and pioneered a joint DIA-ASA scientific working group.

Jesse Berlin received his doctorate in biostatistics from the Harvard School of Public Health in 1988. After 15 years on faculty at the University of Pennsylvania, Jesse left Penn to join Janssen R&D in Biostatistics. He now serves as Vice President of Epidemiology across all of Johnson & Johnson. He has over 270 peer-reviewed publications. He was elected as a fellow of the American Statistical Association in 2004. In 2013, Dr. Berlin received the Lagakos Distinguished Alumni Award from the Department of Biostatistics at the Harvard School of Public Health.
Atul Butte is the Priscilla Chan and Mark Zuckerberg Distinguished Professor and inaugural Director of the Bakar Computational Health Sciences Institute (bchsi.ucsf.edu) at the University of California, San Francisco (UCSF). Dr. Butte is also the Chief Data Scientist for the entire University of California Health System, with 17 health professional schools, 6 medical centers, and 10 hospitals. Dr. Butte has been continually funded by NIH for 20 years, is an inventor on 24 patents, and has authored over 200 publications, with research repeatedly featured in the New York Times, Wall Street Journal, and Wired Magazine. Dr. Butte was elected into the National Academy of Medicine in 2015, and in 2013, he was recognized by the Obama Administration as a White House Champion of Change in Open Science for promoting science through publicly available data. Dr. Butte is also a founder of three investor-backed data-driven companies: Personalis, providing medical genome sequencing services, Carmenta (acquired by Progenity), discovering diagnostics for pregnancy complications, and NuMedii, finding new uses for drugs through open molecular data. Dr. Butte trained in Computer Science at Brown University, worked as a software engineer at Apple and Microsoft, received his MD at Brown University, trained in Pediatrics and Pediatric Endocrinology at Children's Hospital Boston, then received his PhD from Harvard Medical School and MIT.

Jacqueline Corrigan-Curay serves as Director of CDER’s Office of Medical Policy (OMP). She leads the development, coordination, and implementation of medical policy programs and strategic initiatives. She works collaboratively with other CDER program areas, FDA centers, and stakeholders on enhancing policies to improve drug development and regulatory review processes. OMP is comprised of the Office of Prescription Drug Promotion (OPDP) and the Office of Medical Policy Initiatives (OMPI). OPDP oversees the regulation of prescription drug promotion and advertising. OMPI provides oversight and direction for new and ongoing policy initiatives in broad-based medical and clinical policy areas. Prior to joining FDA, she served as supervisory medical officer with the Immediate Office of the Director, National Heart, Lung and Blood Institute (NHLBI), at National Institute of Health’s (NIH) where she focused on developing policies and procedures to enhance the clinical trial enterprise. She also served as the Director of the Office of Biotechnology Activities (OBA), Office of Science Policy at NIH, where she was executive secretary of the NIH Recombinant DNA Advisory Committee. She has held positions as an attending physician with the VA Medical Center, a policy analyst with the Congressional Office of Technology Assessment, and a practicing attorney in Washington, D.C. Dr. Corrigan-Curay earned her law degree from Harvard Law School, her medical degree from University of Maryland School of Medicine, and a bachelor’s degree in history of science from Harvard/Radcliffe College in Cambridge, MA. She completed her training in internal medicine at Georgetown University Medical Center, where she also served as a clinical assistant professor of medicine. She continues to practice internal medicine part-time at the Veterans Affairs Medical Center in Washington, D.C.

Cathy Critchlow leads the Center for Observational Research (CfOR), providing strategic leadership to the design of non-interventional studies to inform decision-making by stakeholders across the healthcare ecosystem. The CfOR Real World Data Platform provides access to patient health data and innovative visualization and analytic tools to aid Development, Regulatory, Medical Affairs, Value & Access and Global Marketing teams in the generation of real world evidence supporting drug development and commercialization. Dr. Critchlow earned her bachelor’s degree from Stanford University, and both a master’s degree in biomathematics and a doctorate in epidemiology from the University of Washington at Seattle.
William H. Crown is Chief Scientific Officer of OptumLabs. In this role, he is responsible for research activities of the Labs. From 2004-2013, Dr. Crown was President of the health economics, late phase research, data products, and epidemiology business units at Optum Life Sciences. He was Vice President of Outcomes Research and Econometrics at Thomson Reuters Medstat from 1994-2004. From 1982-1995, Dr. Crown was a faculty member of the Florence Heller Graduate School, Brandeis University, where he taught graduate courses in statistics and conducted research on the economics of aging and long-term care policy. He received his doctorate degree in urban and regional studies from the Massachusetts Institute of Technology, and a master of arts in economics from Boston University. The author of two books and co-author of two others, Dr. Crown has published over 160 peer-reviewed journal articles, book chapters, and other scholarly papers. Known for his early application of sample selection bias models in the pharmaceutical outcomes research literature, he is a frequent speaker on statistical methods for the analysis of observational data at professional meetings and conferences. In addition to his CSO role within Optum Labs, Dr. Crown is Affiliate Faculty, Mongon Institute for Health Policy, Harvard University. He was also 2013-14 President of the International Society of Pharmacoeconomics and Outcomes Research.

Lesley Curtis, a health services researcher by training, is an expert in the use of Medicare claims data for health services and clinical outcomes research. She has led the linkage of Medicare claims with several large clinical registries and epidemiological cohort studies including the Framingham Heart Study and the Cardiovascular Health Study. Dr. Curtis serves on the American Heart Association/American College of Cardiology Task Force on Practice Guidelines and on the American Heart Association’s Task Force on Performance Measures, working to continuously improve the incorporation of evidence into health care delivery. Additionally, she serves as Co-Lead of the Data Core for the FDA’s Sentinel Initiative, Chair of the Subcommittee on Data Quality for the National Evaluation System for health Technology (NEST) Coordinating Center, Co-PI of the NIH Health Care Systems Collaboratory, and Lead of the Distributed Research Network Operations Center for PCORI’s National Clinical Research Network (PCORnet), working with health systems and patient networks to develop a harmonized data infrastructure for robust observational and interventional research. Dr. Curtis was recently appointed to the National Academies of Sciences, Engineering, and Medicine’s Committee on the Clinical Utility of Treating Patients with Compounded “Bioidentical Hormone Replacement Therapy” and is an inducted fellow in the American College of Medical Informatics.

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.

Nancy Dreyer is Chief Scientific Officer and Senior Vice President at IQVIA. She focuses on generating real-world evidence for regulators, clinicians, patients and payers through pragmatic trials and non-interventional approaches and has more than 200 publications. She is a member of the PCORI Clinical Trials Methods Advisory Panel and has been a Standing Consultant to the National Football League Health & Safety Executive Committee since 2013. She has been named a Fellow of both the Drug Information Association (DIA) and the International Society of Pharmacoepidemiology, and also is an Adjunct Professor of Epidemiology at the Gillings School of Global Public Health at the University of North Carolina. In 2019 she received the DIA Global Inspire Award for Author of the Year for “Advancing a framework for regulatory use of real world evidence: When real is reliable,” an article that was downloaded nearly 7,000 times.

Louis Fiore is a professor of Medicine and Public Health at the Boston University Schools of Medicine and Public Health. He has 25 years of experience in clinical research in the areas of clinical trials, biobanking, epidemiology and informatics as the Executive Director of the Massachusetts Veterans Epidemiology Research and Information Center at the VA Boston Healthcare System. His current focus is on embedding clinical research into the clinical care ecosystem through the Point of Care Clinical Trials Program and is looking to extend that work through creation of methodology to implement randomization for real-world learning in healthcare systems (outside of the context of a clinical trial). He is a proponent of data sharing and strives to reduce silos that isolate researchers from each other and from the clinical care world that they ultimately serve. Together these interests constitute core components of a healthcare system capable of accelerating the pace of discovery for improved care at both the patient and population levels.

Martin Gibson is the Chief Executive of NorthWest EHealth, a company focused on revolutionising the use of electronic health data to support the delivery of clinical trials for the life sciences industry and academia. NWEH were the organisation behind the successful delivery of GSK’s Salford Lung Study – the world’s first real-world large-scale pragmatic randomised clinical trial. Martin is also Clinical Director of NIHR’s Clinical Research Network for Greater Manchester, and clinical lead for business development for the national NIHR clinical research network. He is a Consultant Physician (specialising in diabetes and endocrinology) at Salford Royal NHS Foundation Trust.
**Iris Goetz** graduated in medicine in Germany and worked in paediatric medicine prior to undertaking a research fellowship at Great Ormond Street Hospital, London, UK. Following an MSc in Epidemiology at London School of Hygiene and Tropical Medicine she joined the Global Health Outcomes Research Department of Eli Lilly in the UK in 2001. Her primary focus of work lies in Real World Evidence research including observational studies and randomised RWE (pragmatic) trials with emphasis on methodology and research design across a range of disease areas. She is engaged in internal initiatives dealing with the applications of real world data in various disease areas with focus on Biomedicine and the engagement of respective stakeholders throughout the process. She has been co-leading the Innovative Medicine Initiative (IMI) GetReal 1 and 2 workpackage on ‘Pragmatic Trials’ in collaboration with an academic co-partner from 2013-to date.

**Paul Harris** is professor of biomedical informatics and biomedical engineering with extensive experience working in the field of clinical and translational research informatics. He serves as director of the Vanderbilt University Office of Research Informatics and is very active in the NIH Clinical and Translational Science Award (CTSA) informatics community. In addition to supporting the Vanderbilt University research enterprise, Dr. Harris devised and created REDCap ([www.projectredcap.org](http://www.projectredcap.org)), a data collection toolset that has seen widespread adoption by more than 2600 institutional partners and 635,000 end-users across 116 countries. He also created and runs a national program ([www.researchmatch.org](http://www.researchmatch.org)) designed to match individuals wishing to volunteer for studies and researchers recruiting patients for studies and trials. ResearchMatch is serving over 122,000 research volunteers and 142 research institutions.

**Adrian Hernandez** is a cardiologist with extensive experience in clinical research ranging from clinical trials to outcomes and health services research. Before becoming Vice Dean for Clinical Research in 2017, he was a Faculty Associate Director and Director of Health Services and Outcomes Research at the Duke Clinical Research Institute. He is the Coordinating Center Principal Investigator for multiple networks and clinical trials such as the NHLBI’s Heart Failure Research Network, PCORI’s National Patient-Centered Clinical Research Network (PCORnet) and NIH’s Health System Collaboratory all focused on leveraging new methods to improve clinical research and implementation of evidence into clinical practice.

**Elaine Irving** has over 20 years experience in the pharmaceutical industry spanning early and late stage discovery & development. In her current role, Elaine is part of GSKs Value Evidence and Outcomes group where she leads a group of clinical researchers focussed on the delivery of interventional and non-interventional studies in the routine care setting to support life-cycle management of GSKs medicines. Elaine’s early career focussed on leading the discovery and development of novel assets for the treatment of Alzheimer’s Disease and Stroke during which time she became and expert in translational science and established the first Translational Science function at GSK. Later Elaine became accountable for driving GSKs strategy for leveraging funding through the Innovative Medicines Initiative to increase efficiency of the R&D process. During this time she led negotiations with senior officials within the European Commission to define a joint strategy and implementation framework for enabling the discovery and development of novel antibiotics leveraging the benefits of public private partnership which led to the provision of €450K funding for the New Drugs for Bad Bugs Initiative. She
was also invited by the EFPIA Research Directors Group to lead the development of the €3Billion IMI2 Strategic Research Agenda defining where investment should be focussed to accelerate R&D. Elaine graduated from Edinburgh University, Scotland with a BSc in Pharmacology and completed her PhD in the field of neurodegenerative research at the Wellcome Surgical Institute, University of Glasgow.

Loretta Jacques is Clinical Development Director and SLS Project Leader at GSK. Loretta graduated with a PhD in Biochemistry from University College Cork in Ireland. Thereafter she was employed as a post-doctoral Fellow at Guy’s Hospital in London for a couple of years and then moved to work in clinical development in the pharmaceutical industry. Over her years in clinical development, Loretta worked mainly in the Respiratory and GI areas, working on many assets including Advair and Breo (where she led the global clinical development programme for the asthma indication). Loretta assumed the role of SLS project leader in 2016 taking accountability for delivery of the SLS results and subsequent publications as well as oversight of the work with the NWEH to ensure not only timely delivery of the data but also subsequent decommissioning and archiving by NWEH of the SLS data. Loretta thinks that working on RWE studies such as SLS is hugely exciting but definitely requires an open mindset and a flexible approach whilst at all times ensuring overall quality.

Martin Landray’s work seeks to further understanding of the determinants of common diseases through the design, conduct and analysis of large-scale clinical trials and prospective cohort studies. He leads international trials that have enrolled over 65,000 individuals, producing results that have changed regulatory drug approvals, influenced clinical guidelines and changed prescribing practice. He is heavily involved in efforts to streamline clinical trials, working with regulatory agencies to facilitate efficient and cost-effective trials. He is a member of the Steering Committee of the FDA Clinical Trial Transformation Initiative, leading the Monitoring, Quality by Design and Mobile Clinical Trials projects. He serves as a member of the NHS Digital Research Advisory Group and leads the 21st Century Clinical Trials programme for Health Data Research UK.

Leanne Larson brings over 25 years’ experience in healthcare, featuring extensive work in pharmaceutical product development and commercialization, and in healthcare technology and operational consulting. Leanne is an industry leader in designing and leading observational / non-interventional studies and in advancing the science of outcomes research, and publishes and speaks widely on a variety of topics in this area. Prior to joining PAREXEL, Leanne served as VP for Quintiles/Outcome, ICON, and Sq2 Healthcare Intelligence. She also held positions in Ernst & Young’s Life Sciences consulting practice, and as a Senior Health Science Coordinator (MSL) with Merck and Co. Leanne holds a Master of Health Administration degree from Governors State University and a Bachelor of Science in Community Health from the University of Illinois. She is an Instructor in Health Service Systems at the Keller Graduate School of Management, and is an Invited Reviewer and Adviser for US Centers for Disease Control and Prevention (CDC), as well as a Charter Member of the International Society of Pharmacoeconomics and Outcomes Research (ISPOR).
Lisa LaVange is Professor and Associate Chair of the Department of Biostatistics in the Gillings School of Global Public Health at the University of North Carolina at Chapel Hill. She is also director of the department’s Collaborative Studies Coordinating Center (CSCC), overseeing faculty, staff, and students involved in large-scale clinical trials and epidemiological studies coordinated by the center. From 2011 to 2017, Dr. LaVange was director of the Office of Biostatistics in the United States Food and Drug Administration (FDA) Center for Drug Evaluation and Research (CDER). There, she oversaw more than 200 statisticians and other staff members involved in the development and application of statistical methodology for drug regulation. She was a leader in developing and assessing the effectiveness and appropriateness of innovative statistical methods intended to accelerate the process from drug discovery to clinical trials to FDA approval and patients’ benefit, with a particular focus on rare diseases. Prior to her government and academic experience, she spent 16 years in non-profit research and 10 years in the pharmaceutical industry. Dr. LaVange is an elected fellow of the American Statistical Association (ASA) and was the 2018 ASA President.

Mark Levenson is the Director of the Division of Biometrics 7 in the Office of Biostatistics/Office of Translational Sciences/Center for Drug Evaluation and Research of FDA. At FDA, he has been the primary reviewer or secondary reviewer on many major pre-market and post-market drug safety problems. He has contributed to the methodology of the application of meta-analysis and propensity score analysis to the regulatory setting. He is active in CDER’s efforts in the Sentinel Initiative, reducing prescription opioid abuse, and real-world evidence.

David Madigan is Professor of Statistics at Columbia University in New York City and Dean Emeritus of Arts and Sciences. He received a bachelor’s degree in Mathematical Sciences and a Ph.D. in Statistics, both from Trinity College Dublin. He has previously worked for AT&T Inc., Soliloquy Inc., the University of Washington, Rutgers University, and SkillSoft, Inc. He has over 200 publications in such areas as Bayesian statistics, text mining, Monte Carlo methods, pharmacovigilance and probabilistic graphical models. He is an elected Fellow of the American Statistical Association, the Institute of Mathematical Statistics, and the American Association for the Advancement of Science. He has served terms as Editor-in-Chief of Statistical Science and of Statistical Analysis and Data Mining – the ASA Data Science Journal.

Mark McClellan is the Robert J. Margolis Professor of Business, Medicine, and Health Policy, and founding Director of the Duke-Margolis Center for Health Policy at Duke University. With offices in Durham, NC and Washington, DC, the Duke-Margolis Center is a university-wide, interdisciplinary initiative that is nationally and internationally recognized for its research, evaluation, implementation, and educational initiatives to improve health and health policy. The Center integrates Duke’s expertise in the social, clinical, and analytical sciences with health care leader and stakeholder engagement to develop and apply policy solutions that improve health and the value of health care locally, nationally, and worldwide. Dr. McClellan is a physician and an economist who has informed and improved a wide range of strategies and policy reforms to advance health care, including payment reform to promote better outcomes and lower costs, methods for development and use of real-world evidence, and strategies for more effective biomedical 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. With highly distinguished record in public service and academic research, Dr. McClellan is a former administrator of the Centers for Medicare & Medicaid Services and former commissioner of the U.S. Food and Drug Administration (FDA), where he developed and implemented major reforms in health policy. These reforms 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. He previously served as a member of the President’s Council of Economic Advisers, senior director for health care policy at the White House, and Deputy Assistant Secretary for Economic Policy at the Department of the Treasury. Dr. McClellan is the founding chair and a current board member of the Reagan-Udall Foundation for the FDA and a member of the National Academy of Medicine (NAM), where he chairs the 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 is also a Senior Advisor on the faculty of the University of Texas Dell Medical School and co-chair of the Accountable Care Learning Collaborative. Dr. McClellan is an independent director on the boards of Johnson & Johnson, Cigna, and Alignment Healthcare. 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.

Michael O’Neal, the Chief Medical Officer at Bioclinica, provides leadership to all on-staff radiologists and external radiology consultants, manages the organizational planning and operational needs of Bioclinica’s Medical Affairs department, and oversees physician training, service quality, and process improvements. Dr. O’Neal is a graduate of Rutgers University and earned his M.D. at the Universidad Autonoma de Guadalajara. He has over 20 years of experience with extensive clinical training in internal medicine and diagnostic radiology including an imaging fellowship at St. Barnabas Medical Center. In addition to oversight of imaging data in support of regulatory approval for many oncology drugs, Dr. O’Neal has participated in protocol review, charter development, and independent review for nearly 300 clinical trials to date. Dr. O’Neal holds certifications from the American Board of Radiology and the American College of Radiology and is a member of many professional organizations, including the Radiological Society of North America (RSNA), the American College of Radiology (ACR), the American Medical Association (AMA), the Drug Information Association (DIA), the American Society for Clinical Oncology (ASCO), and the American Society of Hematology (ASH). Prior to joining Bioclinica, he served as the Chairman for the Department of Radiology at the Hospital Center at Orange, as the Vice President of Medical Affairs at RadPharm, and as the Chief Medical Officer at Core Lab Partners.
Steven Piantadosi received his medical degree from the University of North Carolina, Chapel Hill, followed by a PhD in Biomathematics at the University of Alabama, Birmingham. After six years as Staff Fellow at the National Cancer Institute (NCI), he joined the Department of Oncology at the Johns Hopkins Medical Institutions as Director of Biostatistics. He rose through the ranks as Professor of Oncology, and also in the Departments of Biostatistics and Epidemiology at Johns Hopkins School of Public Health. In 2007, he moved to Cedars-Sinai Medical Center in Los Angeles, where he served as the first Director of the Samuel Oschin Comprehensive Cancer Institute for 10 years. A leader in the field of clinical trials design and conduct, Dr. Piantadosi is the author of Clinical Trials: A Methodologic Perspective, the definitive textbook on this subject, which is now in its third edition. He is a highly experienced advisor to academia, government and industry, with a broad scope of accomplishment in the cancer clinical research field, including trials testing surgical approaches, optimizing results of bone marrow transplantation, cancer vaccine trials, phase I/II strategies for combining chemotherapy with biologics, design issues in translational research, strategies for accelerating anticancer drug development, and the role of information science in medical research. In addition to joining the Division of Surgical Oncology as Associate Senior Biostatistician, Dr. Piantadosi also joins the Alliance Executive Committee as the Associate Group Chair for Strategic Initiatives and Innovation as well as the Alliance Statistics and Data Management Program.

David Price is founder and head of The Observational and Pragmatic Research Institute (Singapore), an independent, research-driven organisation established to cultivate initiatives, provide evidence and drive quality standards within the growing field of real-life, pragmatic and observational research. This dynamic organisation delivers pragmatic clinical trials and real-life database research across multiple geographies, including territories in the United States, Europe and the Asia-Pacific region, such as Singapore, South Korea, Taiwan, The Philippines, New Zealand, Australia, Japan and China. He is also head of Optimum Patient Care (Australia and UK), a sciencebased, social enterprise which focuses on disease registries and supporting care in primary and secondary care led by internationally recognised experts in all areas of respiratory medicine. Professor Price established the Optimum Patient Care Research Database (OPCRD; http://opcrd.co.uk/), one of the largest enhanced healthcare databases holding anonymous data from over 700 general practices and approximately 6.3 million patients in the UK. He currently leads the first International Severe Asthma Registry (ISAR; http://isaregistries.org/) by creating a consortium of world leaders at the forefront of severe asthma research from over 20 countries. His efforts have unified the severe asthma world by creating a vibrant space to exchange ideas and by standardising the data fields across countries, which is enabling data to be pooled and shared. Professor Price is Primary Care Respiratory Society Professor of Primary Care Respiratory Medicine at the University of Aberdeen (UK). He was awarded Fellow of ERS (FERS) in 2016. He is also a member of the World Health Organization (WHO) Allergic Rhinitis and its Impact on Asthma (ARIA) executive committee, the World Allergy Organization (WAO) Committee on Asthma, and the WAO Education Council. Professor Price was the founding president of the Respiratory Effectiveness Group (http://www.effectivenessevaluation.org/), a not-for-profit, investigator-led initiative which uses an international collaborative approach to explore the optimum role of real-life research in informing clinical guidelines and improving patient care. Professor Price completed his medical degree at Cambridge University in 1984. He was previously Affiliate Associate Professor in the Department of General Practice at the University of Adelaide, Australia and held an Honorary Chair at the University of East Anglia, UK. He is extensively involved in respiratory and allergy research; his areas of special interest are the ‘real-life’ effectiveness and cost-effectiveness of interventions, clinical trial design, adherence, and patient attitudes.
to their disease. He has authored more than 490 peer-reviewed publications and is responsible for approximately US$50 million in research and clinical development grants. He is currently Editor-in-Chief of the journal Pragmatic and Observational Research and member of the editorial board of several respiratory journals, including The Lancet Respiratory Medicine.

Rita F. Redberg is a cardiologist and Professor of Medicine at the University of California, San Francisco since 1990 and Core Faculty, Philip R Lee Institute for Health Policy Studies. Dr. Redberg is the Chief Editor of JAMA Internal Medicine (formerly Archives of ) since 2009 and has spearheaded the journal’s new focus on health care reform and “less is more”, which highlights areas of health care with no known benefit and definite risks. Her research interests are in the area of health policy and technology assessment, and how to promote high value care, focusing on high risk medical devices as well as the need for inclusion of women in clinical trials of such devices. She has had a long standing commitment to women and heart disease and cofounded the UCSF Center of Excellence in Women’s Health in 1997. She was honored to receive the Women’s Day Red Dress Award in 2011 for her leadership in the area of heart disease in women and the Bay Area American Heart Association Red Dress Award in 2010. She is a proud member of the Women’s Heart Alliance. Dr. Redberg recently completed a 6 year term on the Medicare Payment Advisory Commission, which advises Congress on Medicare payment issues. She also served on the Medicare Evidence, Development and Coverage Advisory Committee from 2003-2006 and was reappointed as Chairwoman of MEDCAC from 2012 - 2016. Dr. Redberg is a member of the California Technology Assessment Forum, the Medical Policy Technology and Advisory Committee, Blue Cross Blue Shield Medical Advisory Panel and served on the Food and Drug Administration Cardiovascular Devices Expert Panel. She was a member of the American College of Cardiology’s (ACC) Clinical Quality Committee and served on the Quality in Technology Work Group. She chaired the AHA/ACC Writing Group on Primary Prevention Performance Measures. She has given Congressional testimony multiple times in hearings related to the issue of balancing safety and innovation in medical device approvals. Dr. Redberg worked in the office of Senator Hatch and with the Senate Judiciary Committee on FDA-related matters during her tenure as a Robert Wood Johnson Health Policy Fellow, 2003-2006. Dr. Redberg was elected to the National Academy of Medicine (formerly Institute of Medicine, IOM). She was a member of the IOM’s Learning Health Care Committee, which produced the report Best Care at Lower Cost in September 2012 and of the National Academy of Medicine writing group on A Learning System for Military Trauma Care. Dr. Redberg has authored several books, including You Can Be a Woman Cardiologist, Heart Healthy: The Step-by-Step Guide to Preventing and Healing Heart Disease, and Betty Crocker Cookbook for Women: the Complete Guide to Women’s Health and Wellness at Every Stage of Life. She has done hundreds of radio, television and newspaper interviews on health related topics including being featured in The New York Times, Wall Street Journal, USA Today, National Public Radio and the Today Show. Dr. Redberg graduated from Cornell University and the University of Pennsylvania Medical School and has a Master of Science in Health Policy and Administration from the London School of Economics.
Satrajit Roychoudhury is a Senior Director and a member of Statistical Research and Innovation group in Pfizer Inc. Prior to joining, he was a member of Statistical Methodology and Consulting group at Novartis. He started his career as a research statistician in Schering-Plough Research Institute (now Merck Co.). He has 10+ years of extensive experience in working in different phases of clinical trials. His primary expertise includes implementation of innovative statistical methodology in clinical trials. He has co-authored several publications/book chapters in this area and provided statistical training at major conferences. His areas of research include the use of survival analysis, model-informed drug development and Bayesian methods in clinical trials.

Simon Skibsted serves as Director for Clinical Development and Outcomes Research at Novo Nordisk Inc. In this capacity, he has led the cross functional efforts of implementing novel trials designs including pragmatic trials within his organization across various therapeutic areas and development phases. This has led to the initiation of real world randomized pragmatic trials in employer, health system, and payer settings. Prior to this, Dr. Skibsted worked in a global function where he was medically and scientifically responsible for the conduct of pre and post approval randomized trials including large scale cardiovascular outcomes trials enabling a solid experience in planning, designing, executing, and reporting of trials within the setting of diabetes and cardiovascular disease. Dr. Skibsted is particularly focused on ways to translate efficacy to effectiveness. This includes the development and implementation of new tools and processes to assist in assessing value of interventions in settings and populations in which the intervention is intended to be used once it is on the market. Dr. Skibsted earned his MD degree at University of Copenhagen and his PhD degree from Aarhus University. Furthermore, Dr. Skibsted has an MPH degree in clinical effectiveness from Harvard University.

Peter Stein is the Director of CDER’s Office of New Drugs (OND). OND is responsible for the regulatory oversight of investigational studies during drug development and decisions regarding marketing approval for new (innovator or non-generic) drugs, including decisions related to changes to already marketed products. OND provides guidance to regulated industry on a wide variety of clinical, scientific, and regulatory matters. A nationally-recognized leader in pharmaceutical research and development, Dr. Stein joined CDER in 2016 as the OND Deputy Director. Before coming to FDA, he served as Vice President for late stage development, diabetes, and endocrinology at Merck Research Laboratories. He also served as Vice President, head of metabolism development at Janssen. He has more than 30 years of academic, clinical, and industry experience. Dr. Stein holds a bachelor’s degree in history from the University of Rochester in New York and a medical degree from University of Pennsylvania. He trained at Yale University and Yale-New Haven Hospital in internal medicine and in endocrinology and metabolism.

Elizabeth A. Sugar is an Associate Scientist in the Department of Biostatistics at the Bloomberg School of Public Health at Johns Hopkins University. She holds joint appointments at the Center for Clinical Trials, Department of Epidemiology, Bloomberg School of Public Health and at the Division of Biostatistics and Bioinformatics, Sidney Kimmel Comprehensive Cancer Center, School of Medicine, Johns Hopkins University. Her research focuses on the design and analysis of clinical trials and longitudinal studies. She received a PhD from the University of Washington in Seattle in 2003. After graduation, she joined the Division of Biostatistics and Bioinformatics at the Sidney Kimmel Comprehensive Cancer Center as an Instructor. Her work in oncology has focused on translational
studies and early stage (Phase I and II) clinical trials for immunologic therapies. In 2007, she accepted a position as an Assistant Scientist in the Department of Epidemiology. There she expanded her research to include large, multicenter clinical trials and observational studies with a focus on ophthalmology and airways disease. She moved to the Department of Biostatistics in 2012 to take the lead in teaching and mentoring biostatistics students interested in research in clinical trials and was promoted to Associate Scientist in 2014. During the last 16 years, she has assumed a leadership role in the Coordinating Center (e.g. PI, Deputy Director, Lead Statistician) for a number of multicenter studies as well as having served on DSMCs for NIH-sponsored clinical trials.

**Robert Temple** serves as CDER’s Deputy Center Director for Clinical Science and Senior Advisor in the Immediate Office of the Office of New Drugs (OND). As the senior advisor, Bob is a consultant to the OND director and other FDA officials on matters related to clinical program objectives. Dr. Temple received his medical degree from the New York University School of Medicine in 1967. In 1972, he joined CDER as a Medical Officer in the Division of Metabolic and Endocrine Drug Products. He later moved into the position of Director of the Division of Cardio-Renal Drug Products. Before becoming Senior Advisor in OND, Dr. Temple was the Acting Deputy Director of OND’s Office of Drug Evaluation-I (ODE-I) which is responsible for the regulation of cardiovascular and renal, neurology, and psychiatry drug products. He served in this capacity for more than 23 years—since the office’s establishment in 1995. Dr. Temple has a long-standing interest in the design and conduct of clinical trials. He has written extensively on this subject, especially on choice of control group in clinical trials, evaluation and active control trials, trials to evaluate dose-response, and trials using “enrichment” designs. He has been involved in the development of many International Conference on Harmonization (ICH) guidelines and numerous FDA guidances, including ones on study enrichment and on issues related to the design and interpretation of non-inferiority studies.

**Sean Tunis** is the Founder and Senior Strategic Advisor with the Center for Medical Technology Policy in Baltimore, Maryland. CMTP is an independent, non-profit organization that provides a neutral platform for multi-stakeholder collaborations that are focused on improving the quality, relevance, and efficiency of clinical research. Dr. Tunis is the current President of Health Technology Assessment International and a Principal with Rubix Health, where he consults for a number of public and private sector organizations focused on issues of comparative effectiveness, outcomes measurement, innovation, health technology assessment, evidence-based medicine, clinical research, and reimbursement. Through September of 2005, Dr. Tunis was the Director of the Office of Clinical Standards and Quality and Chief Medical Officer at the Centers for Medicare and Medicaid Services (CMS). In this role, he had lead responsibility for clinical policy and quality for the Medicare and Medicaid programs. Dr. Tunis supervised the development of national coverage policies, quality standards for Medicare and Medicaid providers; quality measurement and public reporting initiatives, and the Quality Improvement Organization program. As Chief Medical Officer, Dr. Tunis served as the senior advisor to the CMS Administrator on clinical and scientific policy. He also co-chaired the CMS Council on Technology and Innovation. Dr. Tunis joined CMS in 2000 as the Director of the Coverage and Analysis Group. Before joining CMS, Dr. Tunis was a senior research scientist with the Technology Assessment Group, where his focus was on the design and implementation of prospective comparative effectiveness trials and clinical registries. Dr. Tunis also served as the Director of the Health Program at the Congressional Office of Technology Assessment and as a health policy advisor to the U.S. Senate Committee on Labor and Human Resources, where he participated in policy development regarding pharmaceutical and device regulation.
He received a B.S. degree in Biology and History of Science from the Cornell University School of Agriculture, and a medical degree and masters in Health Services Research from the Stanford University School of Medicine. Dr. Tunis did his residency training at UCLA and the University of Maryland in Emergency Medicine and Internal Medicine. He is board certified in Internal Medicine and holds adjunct faculty appointments at Johns Hopkins, Tufts and the University of California San Francisco Schools of Medicine.

**Ellis Unger** is the Director, Office of Drug Evaluation-I, Office of New Drugs (OND), CDER. His Office oversees the regulation of drugs for cardiovascular, renal, neurological, and psychiatric disorders. Dr. Unger obtained his medical degree from the University of Cincinnati, and he is boarded in internal medicine and cardiovascular diseases, having trained at the Medical College of Virginia and the Johns Hopkins Hospital, respectively. Dr. Unger was a Senior Investigator in the National Heart, Lung, and Blood Institute, NIH, from 1983 to 1997. From 1997 to 2003, Dr. Unger held various positions within CBER. Dr. Unger joined CDER as Deputy Director of the Cardiorenal Division in 2003, and he subsequently became the Director of the Office of Drug Evaluation-I, in July 2012

**Joanne Waldstreicher** is Chief Medical Officer, Johnson & Johnson. In this role, she has oversight across pharmaceuticals, devices and consumer products for safety, epidemiology, clinical and regulatory operations transformation, collaborations on ethical science, and technology and R&D policies, including those related to clinical trial transparency and compassionate access. She chairs the R&D Development Pipeline Review Committee for The Janssen Pharmaceutical Companies of Johnson & Johnson, and supports the Medical Devices and Consumer Development Committees. Joanne is also a faculty affiliate of the Division of Medical Ethics, Department of Population Health, New York University School of Medicine. Among her prior roles, Joanne was responsible for late-stage development in neuroscience, cardiovascular disease and metabolism at Janssen. Before joining Johnson & Johnson in 2002, she headed endocrinology and metabolism clinical research at Merck Research Laboratories, overseeing development programs in atherosclerosis, obesity, diabetes, urology and dermatology. She was honored with the Key Innovator Award, among other distinctions. Joanne received both the Jonas Salk and Belle Zeller scholarships from the City University of New York, and graduated summa cum laude from Brooklyn College. She graduated cum laude from Harvard Medical School, completed her internship and residency at Beth Israel Hospital and her endocrinology fellowship at Massachusetts General Hospital. She has received numerous awards and scholarships, and is an active scientific author. In 2016, the National Association of Female Executives named her Healthcare Champion of the Year. Joanne combines broad experience in science and medicine with a passion for advancing transparency and ethics, with a goal of improving the lives of patients and consumers worldwide.

**Vince Willey** is a Principal Scientist for HealthCore. He is responsible for the overall scientific integrity for specific health economics and outcomes research as well as late phase studies with a particular emphasis on pragmatic clinical trials. His focus is on assuring that HealthCore’s studies are sound from a clinical, research methodology and health care system perspective. From 2014 to 2017, Dr. Willey served as the Staff Vice President, Life Sciences Research for HealthCore. Prior to this position, he was an Associate Professor of Pharmacy and Vice Chair at the Philadelphia College of Pharmacy within the University of the Sciences. This is his second stint with HealthCore, as he was
previously with the company from 1996 to 2008. Dr. Willey’s pharmacy practice experience spans 25+ years in the areas of community pharmacy, long-term care consulting, home infusion, and ambulatory care clinical services. From 2009 to 2014, he developed and implemented a pharmacist-managed program to assist a primary care physician group practice in the management of patients with diabetes, cardiovascular disease, and respiratory disease within a patient-centered medical home. Dr. Willey has published more than 50 original research manuscripts in peer reviewed journals and has presented at numerous medical, pharmacy and health-economic research national meetings. He has also served as a peer reviewer for multiple medical and pharmacy journals and is currently a member of the Editorial Board of the American Health and Drug Benefits journal and formally a member of the Journal of Managed Care Pharmacy’s Editorial Advisory Board. Dr. Willey served as the Chair of the Advisory Group for the American Pharmacists Association that was responsible for the content of their Pharmacy-Based Cardiovascular Disease Risk Management Certificate Training Program, a Member of the Agency for Healthcare Research and Quality’s Comparative Effectiveness Research Pharmacy Workgroup, and was previously a member of the Institutional Review Board at the University of Pennsylvania. Dr. Willey received his Bachelor of Pharmacy degree from the Philadelphia College of Pharmacy and Science and his Doctor of Pharmacy degree from the University of the Sciences in Philadelphia. He currently maintains his credentials as a Board Certified Ambulatory Care pharmacist.

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