Duke | Robert J. Margolis, MD Center for Health Policy

Data Sharing to Accelerate Therapeutic Development for Rare Diseases

Virtual (Zoom)
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Biographies

Jeff Barrett is Senior Advisor at the Critical Path Institute serving as a critical liaison between C-Path and the pharmaceutical industry, foundations, and other key stakeholders, helping grow C-Path's portfolio in drug development solutions, with a focus, but not limited to model-informed drug development (MIDD) and real world data (RWD) technologies. Jeff was the former Head of Quantitative Sciences at the Bill & Melinda Gates Medical Research Institute (MRI). In this role he was responsible for implementing model-based drug development and employing PK/PD modeling, statistics, and clinical trial simulations to advance the discovery and development of new medicines and vaccines to treat malaria, tuberculosis (TB) and enteric diarrheal disease (EDD). In this role, he engaged and led the Global Health ecosystem in the promotion of Open Science policies to create a collaborative environment for data and model sharing.

Prior to MRI, he was Vice President, Global Head of Translational Informatics at Sanofi Pharmaceuticals. He led various aspects of model-based decision-making spanning from drug discovery through commercialization and provided leadership for Sanofi's cloud-based, high-performance computing and "big data" initiatives. Prior to Sanofi, Jeff spent 10+ years at the University of Pennsylvania where he was Professor, Pediatrics and Director, Laboratory for Applied PK/PD at the Children's Hospital of Pennsylvania. Jeff received his B.S. in Chemical Engineering from Drexel University and Ph.D. in Pharmacokinetics from University of Michigan. He has co-authored over 170 manuscripts and has given over 140 invited lectures on PK/PD, clinical pharmacology and pharmacometrics. He is a fellow of ACCP and AAPS and the recipient of numerous honors including ACCP awards for Young Investigator (2002) and Mentorship in Clinical Pharmacology (2007) and the AAPS Award in Clinical Pharmacology and Translational Research (2011). Dr. Barrett was awarded for Exceptional Innovation and Advancing the Discipline of Pharmacometrics at the International Society for Pharmacometrics (2013) and elected ISOP Fellow (2017). He served on the Editorial Boards of the Journal of Pharmacokinetics and Pharmacodynamics and the ASCPT Pharmacometrics & Systems Pharmacology Journal and is currently the co-Specialty Chief Editor of Frontiers in Obstetric and Pediatric Pharmacology Journal. He is currently an active member of the Child Health and Human Development Pediatrics Subcommittee and is a study section reviewer for this committee. He was a past acting chair of the FDA Advisory Committee for Pharmaceutical Science and Clinical Pharmacology and was a voting member of the committee for 8 years.

Ron Bartek is the co-founder and founding president of the Friedreich's Ataxia Research Alliance, a nonprofit organization dedicated to educational and research efforts aimed at developing treatments and cures for Friedreich's ataxia. He also serves on the board of directors of the National Organization for Rare Disorders and on the Data and Safety Monitoring Board of the NCATS-led Rare Diseases Clinical Research Network. Recently, Bartek served as a member of the National Advisory Neurological Disorders and Stroke Council and sat on a panel of patient advocates assembled by the director of the National Institute of Neurological Disorders and Stroke to help develop the Institute's

strategic plan and enhance two-way communication between the patient and scientific communities. His past experience includes serving as the partner and president of a business and technology development, consulting and government affairs firm (Mehl, Griffin & Bartek) and directing the American Friends of the Czech Republic. Bartek's career also includes 20 years of federal executive branch and legislative branch service in defense, foreign policy and intelligence. Bartek graduated from the United States Military Academy at West Point and holds a master's degree in Russian area studies from Georgetown University.

Shrujal Baxi is a Senior Medical Director and Head of Clinical Science at Flatiron Health. Shrujal is a board-certified medical oncologist who focuses on real-world evidence generation at Flatiron. In addition to active involvement in therapeutic clinical trials, Shrujal has used large national cancer registry data to investigate practice patterns in oncology. In addition, she has published on the non-oncologic outcomes associated with cancer including toxicities of treatment. In response to findings, she investigated systems-level solutions to facilitate delivery of comprehensive survivorship care. Shrujal simultaneously received her medical degree from the University of Illinois College of Medicine at Chicago and her masters in public health with an emphasis on health policy and administration from the University of Illinois School of Public Health. She then moved to New York City to complete residency at New York University. After serving as a chief resident, she completed a fellowship in medical oncology at Memorial Sloan Kettering Cancer Center and joined their faculty in 2011, where she specialized in cancers of the head and neck, salivary glands, and thyroid along with non-melanoma cutaneous malignancies.

Atul Bhattaram is a team leader in the Division of Pharmacometrics within Office of Clinical Pharmacology at the U.S. Food and Drug Administration. Dr. Bhattaram's research interests include application of disease progression models towards the design of clinical trials in Parkinson's disease and Duchenne Muscular Dystrophy.

Vanessa Boulanger is the Director of Research at the National Organization for Rare Disorders (NORD). In this role, she leads the strategic development, growth, and implementation of NORD's research and scientific activities. Prior to joining NORD, Vanessa held leadership positions at the Dana-Farber Cancer Institute in the Center for Community-Based Research, the François-Xavier Bagnoud (FXB) Center for Health and Human Rights, and the Harvard T.H. Chan School of Public Health, in addition to a faculty position at Regis College. Vanessa brings over 15 years of experience addressing health and social inequalities from a range of health and development perspectives to her role at NORD. Vanessa holds an MSc degree in Global Health and Population from Harvard University and a BA in International Development and Social Change from Clark University. In 2018, Vanessa was appointed to a three-year term as a member of the Patient-Centered Outcomes Research Institute (PCORI) Advisory Panel on Rare Diseases.

Michelle Campbell is the Senior Clinical Analyst for Stakeholder Engagement and Clinical Outcomes in the Office of Neuroscience, Office of New Drugs (OND) in FDA's Center for Drug Evaluation and Research. Previously, Dr. Campbell was a reviewer on the Clinical Outcome Assessments (COA) Staff and Scientific Coordinator of the COA Qualification Program in OND. Dr. Campbell's focus is in patient-focused drug development and the use of patient experience data in the regulatory setting. Prior to joining FDA, Dr. Campbell spent more than 10 years conducting research in the academic-clinical setting, including five years in a neurology and developmental medicine department. Dr. Campbell earned her BA in Biology from the College of Notre Dame, her MS in Health Science (concentration in Community Health Education) from Towson University and her PhD in Pharmaceutical Health Services Research from the University of Maryland School of Pharmacy.

Karla Childers is the Senior Director of Strategic Projects in the Office of the Chief Medical Officer at Johnson & Johnson. Ms. Childers joined Johnson & Johnson in October 2013 in the Office of the Chief Medical Officer where her primary responsibility has been leading and coordinating various ethics-based, science policy projects. Her longest running responsibility has been the support and coordination of Johnson & Johnson's Clinical Trial Data Transparency Initiative, including the management of the Yale Open Data Access (YODA) Project collaboration. In this role, she has been active in advocacy efforts supporting implementation of responsible clinical trial data sharing, including consultations with various health authorities and engagement with relevant stakeholder groups. Ms. Childers is also responsible for the management and conduct of the J&J Bioethics Committee, which serves as an internal forum to obtain guidance on bioethical questions within J&J. In that capacity, she coordinates the work of the committee, as well as related bioethics activities (educational programs, policy work) within the Office of the Chief Medical Officer.

Before joining J&J, she was an Associate Director in Global Project Management (GPM) in Merck Research Laboratories (MRL), where she managed cross functional drug development teams in various therapeutic areas and stages of development. Prior to joining GPM, Ms. Childers was a bench chemist in MRL's Process Chemistry group. She received her Bachelor of Arts Degree in Chemistry from Indiana University-Purdue University in Indianapolis and a Master of Science in Jurisprudence (MSJ) with a concentration in Health Law from Seton Hall Law School. She is currently pursuing a Master of Bioethics at Columbia University.

Laurie Conklin is the Vice President of Medical and Regulatory Affairs at ReveraGen BioPharma. Dr. Conklin is a board-certified pediatric gastroenterologist with expertise in pediatric inflammatory bowel diseases, pharmacodynamic biomarkers, and pediatric drug development. She has previously served as a Medical Officer in Pediatrics at CDER at the US Food and Drug Administration, and as the Director of Inflammatory Bowel Disease at Children's National Hospital in Washington DC. In 2017, Dr. Conklin joined ReveraGen BioPharma, a small, privately held company developing a novel drug for the treatment of Duchenne muscular dystrophy. At ReveraGen, Dr. Conklin has led biomarker studies, directs Expanded Access and Compassionate Use, and interacts with patient advocacy groups. She is responsible for regulatory writing, grant writing, leading return of individual trial data to participants (an NIH-funded project), and working with academic collaborators

Mads Dalsgaard is Senior Vice President and Global Head of Experimental Medicine and Clinical Development in Lundbeck. An MD by training with experience from neurology and holds a DMSc in neuroscience as well as an MBA from IMD in Switzerland. Within Lundbeck, Mads Dalsgaard is responsible for global Clinical Development from first-in-man to registration and beyond to phase IV. He is a strong advocate of leveraging innovative and technological tools to support and facilitate clinical development, both on scientific and operational aspects such as around digitalization, data science, biomarkers and bioinformatics, to both secure translational planning and optimal development planning and for the benefit of all stakeholders including patients and caregivers. He is furthermore passionate around precompetitive collaboration across institutions and companies to drive R&D productivity among others through data sharing.

Katie Donohue is the Acting Director of the Division of Rare Diseases and Medical Genetics at the U.S. Food and Drug Administration's Center for Drug Evaluation and Research. Dr. Donohue graduated from medical school at Virginia Commonwealth University. She completed her residency in Internal Medicine and fellowship in Allergy & Immunology at Columbia University - New York Presbyterian Hospital. After completing her clinical training, she joined the faculty at the Columbia University College of Physicians and Surgeons. Dr. Donohue completed a Master's Degree in Epidemiology at the Mailman School of Public Health at Columbia University, with a focus on statistics and clinical trial design. Her research focused on asthma epidemiology, leading to publications of her original research in peer-reviewed journals. Dr. Donohue joined the FDA in 2014, as a Medical Officer in the Division of Pulmonary, Allergy, and Rheumatology Products. She joined the Division of Gastroenterology and Inborn Errors of Metabolism as a Clinical Team Lead in 2017. Dr. Donohue oversees programs for both liver and inborn error indications, with a focus on innovative trial designs in rare diseases.

Billy Dunn is the Director of the Division of Neurology Products at the U.S. Food and Drug Administration's Center for Drug Evaluation and Research. The Division of Neurology Products (DNP) regulates and reviews Investigational New Drug (IND) applications and marketing applications for drug and biologic products for the treatment of neurological diseases and conditions, such as Alzheimer's disease, stroke, Parkinson's disease, Huntington's disease, epilepsy, migraine headaches, muscular dystrophy, amyotrophic lateralsclerosis, multiple sclerosis, cerebral palsy, dementia, narcolepsy, Lennox-Gastaut syndrome, and insomnia.

Michael Feolo is a staff scientist at the NIH's National Center for Biotechnology Information, and since 2007 has been the team lead for the NCBI's database of Genotypes and Phenotypes (dbGaP). The dbGaP is a database designed to allow researchers public access to questionnaires, protocols, methods, phenotypes, molecular data and the results of association analyses in whole genome case/control and longitudinal studies of heritable disease. Through his work on dbGaP and service on several trans-NIH data sharing and policy committees, Michael received the NLM's Rodger's Award in 2011, and NIH Director's awards in 2013 and 2014. Prior to leading the dbGaP team, 2000-2007, Michael worked on developing dbSNP, NCBI's database of genetic variation and dbMHC, NCBI's database focused on the variations in the HLA genes located in a region on human

chromosome 6 known as the Major Histocompatibility Complex (MHC). During this time, Michael participated as a member of the International HapMap planning and analysis committees and coordinated the upload of genotype data from HapMap to the dbSNP. Michael received a BS in Molecular Biology, from the University of Utah 1996, was awarded and National Library of Medicine fellowship in 1997, and earned a MS in Medical Informatics, from the University of Utah, Department of Biomedical Informatics in 1999.

Nicole Mayer Hamblett is a Professor of Pediatrics and Adjunct Professor of Biostatistics at the University of Washington, and Co-Executive Director of the Cystic Fibrosis Therapeutics Development Network (CF TDN) Coordinating Center at Seattle Children's Research Institute. She is a biostatistician who has led the design and analysis of numerous clinical studies which have advanced clinical care and outcomes in CF. Dr. Hamblett directs the TDN Consulting Program which partners with industry sponsors to strategize on complex drug development issues relevant to rare diseases. She is currently a principal investigator on therapeutic trials optimizing antibiotic treatment regimens in CF and studies to advance biomarkers supporting therapeutic development for novel CFTR modulator therapies. Dr. Hamblett is a member of the Cystic Fibrosis Foundation's Patient Registry Committee and Clinical Research Advisory Board.

Sam Hume is the Vice President of Data Science at CDISC. He leads the CDISC Data Science team, which collaborates with CDISC staff and stakeholders to develop tools and standards that support clinical and translational data science. Dr. Hume directs delivery of the CDISC Library, our standards metadata repository, co-leads the CDISC Data Exchange Standards team, and leads the CDISC Real World Data efforts. He has 25 years' experience in clinical research informatics, has held a number of senior technology positions in the biopharmaceutical industry, and holds a doctorate in Information Systems.

Matilde Kam is Associate Director of Analytics and Informatics in the Office of Biostatistics (OB) at CDER. She has strategic and oversight responsibility for matters pertaining to analytics and informatics, including data standards, data integrity/data quality, scientific computing and statistical programming activities in the OB. Prior to joining the FDA, Dr. Kam had significant experience in building and overseeing highly effective statistics and analytics groups in the pharmaceutical industry. Dr. Kam is a Fellow of the American Statistical Association (ASA) and has served in various leadership roles for the Association. She received her PhD in Statistics from the Pennsylvania State University.

Petra Kaufmann is the Senior Vice President of Clinical Development and Translational Medicine at AveXis, a Novartis company. Before joining AveXis, Dr. Kaufmann has held several leadership positions at the US National Institutes of Health, most recently directing the Office of Rare Diseases Research (ORDR). Prior to that, Dr. Kaufmann directed the Division of Clinical Innovation at the National Center for Advancing Translational Sciences, and Clinical Research at the National Institute of Neurological Disorders and Stroke.

Dr. Kaufmann is a neurologist and physician scientist who has spent most of her career at Columbia University where she completed her clinical training, and became a tenured faculty member with research focus on rare diseases. Dr. Kaufmann has served on scientific advisory boards of national and international organizations, and her research has resulted in over 130 publications. A native of Germany, Dr, Kaufmann earned her MD from the University of Bonn and her Masters in Biostatistics from Columbia University's Mailman School of Public Health. Dr. Kaufmann has received board certification in neurology and neuromuscular medicine.

Eileen C. King is a Research Professor within the Division of Biostatistics and Epidemiology at Cincinnati Children's Hospital Medical Center. Dr. King serves as PI on two NIH funded administrative and data coordination research networks: 1) the Data Management and Coordinating Center (DMCC) for the Rare Disease Clinical Research Network (RDCRN); and 2) Administrative Coordinating Center (ACC) for the Bench to Bassinet Program (B2B). Dr. King is also Director of the Data Coordinating Center (DCC) for the Pediatric Obesity Weight Evaluation Registry (POWER) and played a key role in its initiation and development. Dr. King has 11 years of experience leading statistics and data coordination teams in the academic sector in addition to 20 years of industry experience in pharmaceutical and health care research where she was head of the Biometrics and Statistical Sciences Department at The Procter & Gamble Company. Dr. King received her PhD in statistics from Texas A&M University and her MS in statistics from the University of Wyoming and is a Fellow of the American Statistical Association.

Jane Larkindale is the Executive Director of both the Rare Disease Cures Accelerator-Data and Analytics Platform (RDCA-DAP) and the Duchenne Regulatory Science Consortium (D-RSC) at the Critical Path Institute, and runs several other rare disease projects. She has dedicated the past decade to accelerating drug development for rare diseases, through promoting increased efficiency, increased cooperation, shared infrastructure and supporting high quality science. She launched the RDCA-DAP with colleagues from the Food and Drug Administration and the National Organization for Rare Disorders in September of 2019 with the goal of aggregating data across rare diseases to inform on natural history, biomarkers and outcome measures. Through D-RSC, she leads an international consortium dedicated to developing regulatory-ready drug development tools for Duchenne Muscular Dystrophy, specifically developing a clinical trial simulation tool, data standards and an integrated database of clinical data that can be used by the community. She has also worked with several non-profit organizations to support research, develop infrastructure and helps researchers source the tools and collaborators they need. She is a molecular biologist by training, having completed her D.Phil. (Ph.D.) in the Department of Plant Sciences at Oxford University in 2001, which she attended on a Rhodes Scholarship.

Rebecca Li is the Executive Director of Vivli and on faculty at the Center for Bioethics at the Harvard Medical School. Previous to her current role she was the Executive Director of the MRCT Center of Brigham and Women's Hospital and Harvard for over 5 years and remains a Senior Advisor at the Center. She has over 25 years of experience spanning the entire drug development process with experience in Biotech, Pharma and CRO environments. She completed a Fellowship in 2013 in the Division of Medical Ethics at Harvard Medical School. She earned her PhD in Chemical and Biomolecular Engineering from Johns Hopkins University.

Mark McClellan is Director of the Margolis Center for Health Policy at Duke University and the Robert J. Margolis Professor of Business, Medicine, and Policy. He is a physician economist who focuses on quality and value in health care including payment reform, real-world evidence and more effective drug and device innovation. He is former administrator of the Centers for Medicare & Medicaid Services and former commissioner of the U.S. Food and Drug Administration, where he developed and implemented major reforms in health policy. He was previously Senior Fellow at the Brookings Institution and a faculty member at Stanford University.

Theresa Mullin is the CDER Associate Director for Strategic Initiatives at the U.S. Food and Drug Administration. She oversees areas of strategic interest to both the Center and external stakeholders. She leads a variety of CDER efforts including the FDA Patient-Focused Drug Development (PFDD) initiative, which includes work related to the FDA Reauthorization Act (FDARA) and implementation of the 21st Century Cures Act. She also leads CDER's International Program, including the FDA delegation to the International Council on Harmonization (ICH), where she led recent reforms to expand ICH global regulatory membership, and is currently Chair of the ICH Management Committee.

Dr. Mullin previously served as director of CDER's Office of Strategic Program (OSP) for almost a decade. Under her leadership, the office became a critical part of CDER's sustained effort to modernize drug regulatory operations. She led FDA negotiations with industry and public consultations to support the 2017 reauthorization of the Prescription Drug User Fee Act (PDUFA) and Biosimilar User Fee Act and led the previous 3 cycles of negotiation for the 2002, 2007 and 2012 reauthorizations of PDUFA, now providing $1B in annual funding. Before joining CDER in 2007, Dr. Mullin was Assistant Commissioner for Planning in FDA's Office of the Commissioner.

Dr. Mullin received the Senior Executive Service Presidential Rank Award for Distinguished Service in 2011, Presidential Rank Award for Meritorious Service in 2006, and the FDLI Distinguished Service and Leadership Award in 2017. She received her bachelor's degree., magna cum laude, in economics from Boston College, and she has a Ph.D. in public policy analysis from Carnegie-Mellon University.

Anne Pariser is the director of the Office of Rare Diseases Research (ORDR) at the National Center for Advancing Translational Sciences (NCATS) NIH. ORDR is dedicated to accelerating rare diseases research to benefit patients, through rare diseases programs such as the Rare Diseases Clinical Research Network, Genetic and Rare Diseases Information Center (GARD), and the NCATS Toolkit for Patient-focused Therapy Development. Important translational science research initiatives for rare diseases at ORDR include establishing best practices and tools for good quality natural history studies, data standards and sharing initiatives, the development of diagnostic support tools, and rare diseases therapeutics development, as well as translational and basic science research grants and collaborative programs. Dr. Pariser came to NCATS in 2017, and before this, she worked for 16 years at the US Food and Drug Administration Center for Drug Evaluation and Research, where she founded the Rare Diseases Program in FDA CDER's Office of New Drugs in 2010 and served as a Medical Officer and Team Leader for rare diseases drug and biologics product development, review and regulation. Dr. Pariser has 20 years of experience in rare diseases research, and her current research interests include "many diseases at a time" research approaches, such as platforms for gene therapies and other rare disease product development, and informatics approaches to diagnosis.

Klaus Romero is the Chief Science Officer at the Critical Path Institute (C-Path). Dr. Romero, a clinical pharmacologist and epidemiologist with more than 17 years combined experience in academic and pharmaceutical clinical research, translational sciences, pharmacometrics, modeling and simulation and pharmacoepidemiology, has been with C-Path since December 2007. During his tenure with C-Path, he has helped lead clinical pharmacology, pharmacoepidemiology and modeling and simulation projects in Alzheimer's disease, polycystic kidney disease (PKD), tuberculosis, type 1 diabetes, Parkinson's disease, Duchenne muscular dystrophy, kidney transplantation, Huntington's disease and cardiovascular drug safety. His work has helped to achieve major milestones, including the first regulatory endorsement by the U.S. Food and Drug Administration and European Medicines Agency of a clinical trial simulation tool for mild and moderate Alzheimer's disease and the regulatory qualification of the first imaging biomarker for PKD. Dr. Romero's scientific production with C-Path has resulted in more than 60 peer-reviewed publications related to his work with the Institute. Dr. Romero is a fellow of the American College of Clinical Pharmacology, a founding member of the International Society of Pharmacometrics, as well as a member of the American Society for Clinical Pharmacology and Therapeutics, and the International Society for Pharmacoepidemiology. In addition to his duties at C-Path, he serves as Chairman of the Board of Directors for CredibleMeds. Dr. Romero is also a Research Associate Professor at the University of Arizona College of Medicine, Adjunct Professor at the College of Health Solutions at Arizona State University, Adjunct Professor at the University of Southern California's School of Pharmacy and serves on the Scientific Board of Pharos Dx. Dr. Romero received his medical degree from Pontifical Xavierian University, completed his training in Clinical Pharmacology at Columbia National University and holds an MS degree in Epidemiology from the Columbia School of Medicine.

Patroula Smpokou is the Acting Deputy Division Director in the Division of Rare Diseases and Medical Genetics within the Office of New Drugs at the U.S. Food and Drug Administration's Center for Drug Evaluation and Research. Center for Drug Evaluation and Research. In that capacity, she leads multidisciplinary teams in the scientific and regulatory evaluation of products developed for rare biochemical genetic diseases known as inborn errors of metabolism. She has participated in the regulatory review and approval of drugs and biologics for the treatment of various inborn errors of metabolism and has engaged in policy and guidance development in rare diseases. As part of her clinical role at FDA, she has actively engaged with outside stakeholder groups, include academia and patient groups, to facilitate and strengthen collaborations and promote the development of novel therapeutics for rare inherited diseases. She received her medical degree from the University of South Florida and completed her pediatric residency at Yale-New Haven Children's Hospital and fellowship in clinical genetics & genomics at Harvard Medical School. She is board certified in general pediatrics and clinical genetics and genomics and previously practiced clinical and biochemical genetics at Children's National Hospital in Washington, DC. Her areas of interest and expertise include rare genetic diseases of childhood and inborn errors of metabolism.

Tiina Urv is the program director for the Rare Diseases Clinical Research Network (RDCRN), a multidisciplinary international program in the Office of Rare Diseases Research (ORDR). As the lead for the RDCRN program, Tiina collaborates with 10 NIH Institutes to manage 22 consortia and a central Data Management Coordinating Center(link is external) The RDCRN has more than 200 participating sites in 17 countries and more than 100 Patient Advocacy Groups as research partners and conducts research on about 200 rare diseases. Before joining the ORDR, Tiina was a program director in the Division of Clinical Innovation where she provided stewardship for multiple Clinical and Translational Science Awards Program hubs and worked with the Trial Innovation Network as well as NCATS' ORDR. Tiina came to the National Institutes of Health (NIH) in October 2006, working as a program director at the Eunice Kennedy Shriver National Institute of Child Health and Human Development (NICHD) in the Intellectual and Developmental Disabilities Branch. Prior to joining NIH, Tiina studied aging and Alzheimer's Disease in adults with Down syndrome. She was an assistant professor at the University of Massachusetts Medical School's Eunice Kennedy Shriver Center and a research scientist at the New York State Institute for Basic Research in Developmental Disabilities. At NICHD, Tiina coordinated the Hunter Kelly Newborn Screening Research Program, chaired the trans-NIH Fragile X research program, and managed a diverse portfolio of basic, behavioral and bio-behavioral research related to developmental disabilities and rare diseases. Tiina is a developmental disabilities specialist with a Ph.D. from Columbia University. She earned her undergraduate degree from the University of Washington.

James M. Wilson is a Professor in the Perelman School of Medicine at the University of Pennsylvania where he has led an effort to develop the field of gene therapy. Dr. Wilson began his work in gene therapy during his graduate studies at the University of Michigan nearly 40 years ago. He then moved to Boston to do a residency in Internal Medicine at the Massachusetts General Hospital and continued his work in gene therapy at MIT. Dr. Wilson has been at the nexus of this emerging therapeutic area from its birth. He created the first and largest academic-based program in gene therapy after being recruited to Penn in 1993. He initially focused on the clinical translation of existing gene transfer technologies but soon redirected his efforts to the development of second and third generation gene transfer platforms.

His laboratory discovered a family of viruses from primates called adeno-associated viruses (AAV) that could be engineered to be very effective gene transfer vehicles. These so called "vectors" have become the technology platform of choice and have set the stage for the recent resurgence of the field of gene therapy. Dr. Wilson has also been active in facilitating the commercial development of these new gene therapy platforms through the establishment of several biotechnology companies. He is currently leading a national dialogue on the challenges of commercializing these potentially lifesaving treatments due to the disruptive nature they will have on traditional business models. Throughout his career, the focus of Dr. Wilson's research has been rare inherited diseases, ranging from cystic fibrosis to dyslipidemias to a variety of neurologic disorders.

Dr. Wilson has published over 580 papers, reviews, commentaries and editorials in the peer-reviewed literature and is an inventor on over 153 U.S. patents and patent applications and over 200 total issued patents worldwide. He was the second President of the American Society of Gene Therapy. Dr. Wilson was the 2014 recipient of the William Osler Patient Oriented Research Award of the University of Pennsylvania and received the 2015 Scientific Achievement Award from Pennsylvania Bio. Dr. Wilson was noted by the journal Nature Biotechnology to be the "second most productive bio-entrepreneur in life sciences." Dr. Wilson was a long standing Trustee at Albion College where he and his wife established the Lisa and James Wilson Institute for Medicine. He is a founder of a 501(c)3 called Health Through Fitness in Orphan Diseases that supports a bicycle team called Rare Disease Cycling, whose participants compete at a national level and help raise money for rare disease research.

Ian Winburn is the Global Medical Team Lead for Haemophilia, Endocrine, In-born errors of metabolism and Transplantation at Pfizer. Dr Winburn has a medical background in Surgery with a special interest in Renal Transplantation. Previously, Dr Winburn practised clinically in both the UK and New Zealand and completed a PhD at the University of Otago that centred on novel drug discovery in the context of renal ischaemia reperfusion injury. In 2010, he started work for Pfizer in medical affairs within the field of Inflammation. After four successful years in a variety of roles in the UK, Dr Winburn became the European Medical Team Lead for Haemophilia and Transplantation, and subsequently the Western European medical lead for Pfizer's rare disease portfolio. Ian also has worked recently in Japan, leading the Japanese Rare disease medical team. He continues to enjoy bringing together science with clinical practise and is proud to be working to improve the lives of people with haemophilia.

Marta E. Wosińska is the Deputy Director, Policy of the Margolis Center for Health Policy at Duke University and Consulting Professor at the Fuqua School of Business. Widely recognized as an expert on health policy, economics, and regulation, Dr. Wosińska leads the Center's Washington, DC office. In her role, she works with Duke-Margolis leadership on developing the Center's strategy and then executes it with support of the roughly 30-person research team based in DC. Dr. Wosińska's experience spans both academia as well as the executive and legislative branches of the federal government. In 2019, Dr. Wosińska served as an economic advisor to the U.S. Senate Finance Committee, providing drug market analysis and expert guidance for the Committee's bipartisan investigative and legislative work on drug pricing. Dr. Wosińska also served for over three years as Chief Healthcare Economist in the Office of Inspector General (OIG) at the US Department of Health and Human Services. Prior to OIG, Dr. Wosińska had a seven-year tenure at the US Food and Drug Administration (FDA) where she headed the Economics Staff at the Office of Strategic Programs in the Center for Drug Evaluation and Research and served as Senior Economic Advisor to FDA's Deputy Commissioner for Medical Products and Tobacco, in both roles advising senior FDA leadership on a wide range of economic issues related to drugs and biologics. Before entering public service, Dr. Wosińska was an Assistant Professor of Marketing at the Harvard Business School, where her academic research focused on prescription drug marketing. She also was a visiting Assistant Professor at the Columbia Business School, where she developed and taught Healthcare Marketing and Marketing of Pharmaceuticals and Medical Devices. Dr. Wosińska received her PhD in economics from University of California at Berkeley and a bachelor's degree from Arizona State University.

Dina Zand is a medical officer in the Division of Rare Diseases and Medical Genetics at the U.S. Food and Drug Administration's Center for Drug Evaluation and Research. She received both her undergraduate and medical degrees from Northwestern University and completed her residency in pediatrics at St Louis Children's Hospital. She completed additional training in clinical genetics training at the Children's Hospital of Philadelphia. Prior to joining the FDA she held academic positions at Washington University in St. Louis and Children's National Medical Center/George Washington University. Her academic work has resulted in both publications in basic science and rare disease clinical practice

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