

SPEAKER BIOGRAPHIES

SESSION 1 | BEYOND THE TOWER OF BABEL: FDA LEADERSHIP ON DATA STANDARDS



Tracy Dixon-Salazar, PhD

Dr. Dixon-Salazar is a neuroscientist, geneticist, and, patient advocate. Her desire to get her Ph.D. was inspired by her daughter who developed Lennox-Gastaut Syndrome (LGS) at the age of 2.

She did her Ph.D. and post-doctoral work at UC, San Diego where she studied the mechanisms of brain development and synaptic plasticity, identified genetic causes of rare disorders in children, and researched precision therapeutics in stem cell and animal models of pediatric disease.

During her research tenure, and after 16 years of watching daily, unrelenting seizures in her child, she uncovered the driver of her daughter's illness and identified a novel precision therapy that improved her child's life.

Dr. Dixon-Salazar left academia after 15 years in the lab with a drive to decrease the amount of time it would take for patients to benefit from lab discoveries in epilepsy precision medicine. She worked as Associate Research Director at CURE Epilepsy helping to move funding forward for novel translational research in genomic medicine for patients with severe forms of pediatric onset epilepsy. And she now works as Director of Research and Strategy at the Lennox-Gastaut Syndrome (LGS) Foundation where she works directly with patients to access precision therapies and partners with researchers, clinicians, regulators, policy makers, therapy developers, and other advocacy groups in an effort to build a sustainable precision medicine infrastructure for all patients with epilepsy.



Elizabeth Hart, MD

Dr. Elizabeth Hart is the branch chief of General Medicine Branch 1 in the Office of Tissue and Advanced Therapies in the Center for Biologics Evaluation and Research (CBER) at the Food and Drug Administration (FDA). She completed her undergraduate medical training at the University of Pennsylvania, a residency in pediatrics at Rainbow Babies and Children's Hospital, and a fellowship in pediatric endocrinology at Boston Children's Hospital. Prior to joining the FDA in 2014, she practiced clinically and conducted genetic bench research and clinical research in rare pediatric diseases.

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Craig Lipset

Craig Lipset is a recognized leader at the forefront of innovation in clinical research and medicine development. He is the founder of Clinical Innovation Partners, working as an advisor and board member with pharma, tech and venture capital to bring vision and driving action at the intersection of research, digital solutions, and patient engagement. Craig was the Head of Clinical Innovation and Venture Partner at Pfizer, on the founding Operations Committee for TransCelerate Biopharma, and on the founding management teams for two successful startup ventures.

Craig is Adjunct Assistant Professor in Health Informatics at Rutgers University, and Adjunct Instructor in the Center for Health + Technology at University of Rochester. He serves on the Board of Directors for the Foundation for Sarcoidosis Research and the MedStar Health Research Institute, as well as on the Editorial Board for Therapeutic Innovation & Regulatory Science.

Craig has been listed among the PharmaVOICE most inspiring people in the life sciences (Red Jacket hall-of-fame), Pharmaceutical Executive's Emerging Leaders, CenterWatch Top 20 Innovators in Clinical Trials, and the AlleyWatch Who's Who in eHealth.



Anne R. Pariser, MD

Anne Pariser, M.D. 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.

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Anthony Philippakis, MD, PhD

Anthony Philippakis is the Chief Data Officer of the Broad Institute of MIT and Harvard, where he is also an institute scientist.

Philippakis is committed to bridging the gap between data sciences and medicine. He is a cardiologist at Brigham and Women's Hospital, where his primary focus is caring for patients with rare genetic cardiovascular diseases. At the Broad Institute he directs the Data Sciences Platform, an organization of over 100 software engineers and computational biologists that develops software for analyzing genomic and clinical data. In addition to his roles at the Broad Institute and Brigham and Women's Hospital, Philippakis is a Venture Partner at GV, focusing on machine learning, distributed computing, and genomics.

Philippakis received his M.D. from Harvard Medical School and completed a Ph.D. in biophysics at Harvard. As an undergraduate, he studied mathematics at Yale University, and later completed the Part III (equivalent to M.Phil) in mathematics at Cambridge University.



Eric Sid, MD, MHA

Eric Sid joined NCATS as a Presidential Management Fellow (PMF) in the Office of Rare Diseases Research (ORDR) in September 2017. He since has become a program officer in ORDR, where he is the lead for the Genetic and Rare Diseases (GARD) Information Center. GARD provides free, comprehensive, plain-language information on rare and genetic diseases to the public and is accessible through both GARD's website and contact center. Sid is the lead for the Rare Diseases Registry (RaDaR) program, which offers guidance for establishing and maintaining patient registries. He also oversees the NCATS Toolkit for Patient-Focused Therapy Development, which disseminates best practices for patient-partnered research through collaborations between patients and caregivers, community organizations, researchers and NIH/U.S. Food and Drug Administration staff. He has worked to modernize the services and resources offered by these programs through participation in innovation initiatives from the Department of Health and Human Services (HHS), including the HHS Ignite Accelerator and HHS Data Science Co-Lab. Sid has served on scientific committees that address clinical data standards, informed consent for international researchers and public health systems research.

Sid received his M.D. and M.H.A. degrees from the University of Washington's School of Medicine and School of Public Health, respectively. In 2019, he completed his Presidential Management Fellowship, which included a rotation with the VA Center for the Study of Healthcare Innovation, Implementation and Policy of the VA Greater Los Angeles Healthcare System. The PMF program is the premier leadership development program for the federal government.

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**Suzanne Thornton-Jones,
PhD**

Suzanne Thornton-Jones has a Ph.D. in Pharmacology and Toxicology from the Medical College of Virginia/Virginia Commonwealth University. In her current role GTP, she is Chief Regulatory and Compliance Officer. Her primary responsibilities include development and implementation of the regulatory strategy for engagement of Health Authorities around the world throughout the nonclinical research, clinical trial, and registration processes. In addition to her regulatory responsibilities, she also leads the Compliance group, Good Laboratory Practice (GLP) Quality Assurance Unit, which is responsible for auditing nonclinical studies used to support Health Authority regulatory filings. Suzanne has worked in Regulatory Affairs for 15 years in the Pharmaceutical industry, and 6 years as a pharmacology/toxicology reviewer at the Food and Drug Administration, Center for Drug Evaluation and Research (FDA/CDER). Suzanne is the author of 9 peer-reviewed scientific publications and enjoys teaching pharmacology/toxicology regulatory basics at many of the Regulatory Affairs Professional Society (RAPS) training courses.

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SPEAKER BIOGRAPHIES

SESSION 2 | LET'S STOP REINVENTING THE WHEEL: SCALING BEST PRACTICES FOR PATIENT REGISTRIES



Betsy Bogard

Betsy Bogard works in the rare disease community to enable development of transformative therapies. She is currently working in early development at AVROBIO, a biotechnology company based in Cambridge, MA developing gene therapy as a potential one-time treatment option for rare diseases. She serves on the board of directors for RARE-X, a collaborative platform for data sharing in rare diseases. She also chairs the Research Committee for the International FOP Association, a non-profit patient organization for the rare disease fibrodysplasia ossificans progressiva (FOP). Ms. Bogard has nearly 20 years of experience in biotechnology in areas that include program leadership, portfolio management, real-world evidence, registries, health economics, and patient engagement. She has a masters degree in health policy and management from the Harvard School of Public Health. Ms. Bogard lives in Somerville, MA with her two sons.



**David Fajgenbaum, MD,
MBA, MSc**

David Fajgenbaum, MD, MBA, MSc, is an Assistant Professor of Medicine at the University of Pennsylvania, Associate Director of Patient Impact for the Penn Orphan Disease Center, Founding Director of the Center for Cytokine Storm Treatment & Laboratory (CSTL), and Executive Director of the Castleman Disease Collaborative Network (CDCN). He is also the national bestselling author of 'Chasing My Cure: A Doctor's Race to Turn Hope Into Action' and a patient battling idiopathic multicentric Castleman disease (iMCD). He is in his longest remission ever thanks to a precision treatment that he identified, which had never been used before for iMCD. One of the youngest individuals ever appointed to the faculty at Penn Medicine and in the top 1 percent youngest awardees of an NIH R01grant, Dr. Fajgenbaum leads 18 translational research studies at Penn, including a natural history registry that is meeting an EMA post-approval requirement and a clinical trial of the drug that is saving his life. The innovative approach to research that he has spearheaded through the CDCN—the Collaborative Network approach—has been highlighted as a model for rare disease research in NEJM, Journal of Clinical Investigation, and Science. Dr. Fajgenbaum has been profiled in a cover story by The New York Times as well as by Good Morning America, CNN, Forbes 30 Under 30, and the Today Show. Dr. Fajgenbaum received his BS from Georgetown University, MSc in Public Health from the University of Oxford, MD from the University of Pennsylvania, and MBA from The Wharton School.

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Jane Larkindale, DPhil

Jane Larkindale, D.Phil. 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.



**Andrew E. Mulberg, MD,
FAAP**

Andrew is currently an Executive pharmaceutical leader leading strategy and operational aspects of drug and biological development for NDA and BLA submissions for pediatric and adult rare diseases, cystic fibrosis, gastroenterology and hepatology. Formerly, he served Senior Vice President, Global Regulatory Affairs at Amicus Therapeutics from 2016-2020 and responsible for the approval of Galafold (migalastat) for the treatment of Fabry disease in adults. He has been involved in the registration planning for AT-GAA, a novel enzyme replacement therapy for Pompe disease in infants, children and adults. He served as Division Deputy Director of Gastroenterology and Inborn Errors Products, Center for Drug Evaluation and Research (CDER), U.S. Food and Drug Administration (FDA) since 2010-2016. Before joining FDA, Andrew was Portfolio Leader in Established Products responsible for worldwide leadership of Internal Medicine products in Johnson and Johnson from 2000-2010. Andrew is a graduate of Columbia College of Columbia University and of the Mount Sinai School of Medicine. He completed his residency in Pediatrics at the Children's Hospital of Philadelphia followed by a Pediatric Gastroenterology Clinical Fellowship and a Post-Doctoral Fellowship in Cellular and Molecular Physiology at New England Medical Center. Andrew is Adjunct Professor of Pediatrics at the University of Maryland School of Medicine and has served as Attending, Pediatric Gastroenterology and Nutrition at Cooper University Hospital in New Jersey caring for children with gastrointestinal diseases. He is Principal Editor of Pediatric Drug Development: Concepts and Applications published in 2011 and 2013. He is a member of Alpha Omega Alpha Honor Medical Society, American Gastroenterological Association and the North American Society for Pediatric Gastroenterology and Nutrition.

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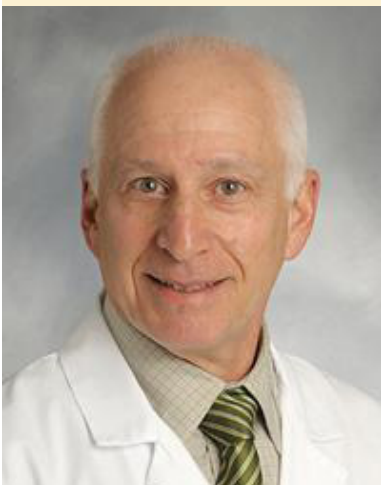


Laura Schanberg, MD

Dr. Schanberg is Professor of Pediatric Rheumatology at Duke Children's Health Center and the Duke Clinical Research Institute (DCRI). She has treated an array of autoimmune conditions and illnesses in children, including inflammatory arthritis, lupus, inflammatory disease of the muscles and vasculitis; however, she has spent most of her professional time doing clinical research funded by NIAMS, PCORI, and various foundations.

Dr. Schanberg is actively engaged with the Childhood Arthritis and Rheumatology Research Association (CARRA), a network aimed at facilitating clinical research in pediatric rheumatology through collaboration. She was part of the founding Steering committee when CARRA began in 2002 and has served in multiple leadership positions including as Chair. Dr. Schanberg is co-PI of the CARRA Registry which was initially funded by the NIH as part of the American Recovery Act, but is now supported by industry and the Arthritis Foundation. The CARRA Registry now includes over 70 CARRA sites and has enrolled over 10,000 children with pediatric rheumatic disease since 2015.

Dr. Schanberg has lead multiple types of research studies including industry funded clinical trials, investigator initiated randomized controlled trials, observational studies PRO validation studies and comparative effective studies. Currently she is leading several clinical research efforts including both industry and investigator initiated clinical trials embedded within the CARRA Registry.



Eric Zuckerman, DO

Eric Zuckerman, D.O. is a Michigan Ophthalmologist, IBD parent and Board Chair of the Pediatric IBD Foundation, a non profit dedicated to improving the lives of children with Crohn's disease and ulcerative colitis through research, advocacy and education. Dr. Zuckerman is also an FDA patient representative in pediatric IBD. In 2015, he presented the model for a data sharing safety registry to meet regulatory requirements, the Children's REgistry for the Advancement of ThErapeutics (CREATE) at FDA's GREAT 3 meeting.

The Foundation continues to work diligently with federal agencies, industry representatives, clinical researchers and professional organizations to develop CREATE, designed to meet phase 4 requirements for pediatric labeling. Currently it takes an average of 9+ years for medications approved in adults to be approved in children and sadly, more children die from off label use in IBD than in any other disease.

The registry is envisioned to follow children longitudinally throughout their lives and will be an indispensable resource, supported by public-private partnerships. The CREATE registry will be accessible to industry, investigators and regulators to facilitate pediatric drug development.

Through the Foundation's web site, Dr. Zuckerman regularly communicates with IBD parents globally. He frequently communicates with IBD parents with educational information and resources, understanding from his own experience the challenges faced by families living with IBD.

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SESSION 3 | LET'S GET REAL (WORLD EVIDENCE): APPLICATIONS FROM THE ONCOLOGY COMMUNITY



Brian Alexander, MD, MPH

Brian Alexander, MD, MPH, is Chief Medical Officer at Foundation Medicine, Inc. and an Associate Professor of Radiation Oncology at Harvard Medical School, the Dana-Farber Cancer Institute/Brigham and Women's Cancer Center. Brian also served as the Head of the Program for Regulatory Science at the Dana-Farber Cancer Institute and the Director of the Harvard/MIT Center for Regulatory Science.

Brian received his BA from Kalamazoo College, MD from the University of Michigan Medical School, and MPH from the Harvard School of Public Health. He completed his training in radiation oncology at the Harvard Radiation Oncology Program. During his residency, he published a book on the use of Bayesian approaches to clinical decision-making and his work applying such approaches to clinical trial designs was supported by a Burroughs-Wellcome Innovations in Regulatory Science Award. His research has resulted in new clinical trial paradigms and transformed the way that data is used in trial design and analysis. Brian co-leads the Adaptive Platform Trials Coalition, a collection of researchers running novel clinical trial inside and outside of oncology, and is Chair of the FDA/Project Datasphere Task Force for the development of external control arms to make clinical research more efficient by leveraging prior clinical trial data.

Brian was also the Principal Investigator and sponsor of INSIGHt, a multi-institutional genomic biomarker-based, Bayesian adaptively randomized platform trial for patients with glioblastoma that he developed with investigators from DFCI. He also co-founded the Global Coalition for Adaptive Research (GCAR) to facilitate the construction of learning systems to accelerate development of new therapies and biomarkers and served as President and Chief Executive Officer. GCAR is the sponsor of the GBM AGILE global clinical trial currently in development and is the subject of a case study at Harvard Business School.

Prior to his faculty appointment at HMS, Brian was a White House Fellow and Special Assistant to the Secretary of Veterans Affairs. In that role, he helped prepare VA for the transition of administrations, worked to develop a public reporting system for quality, and served as a health policy advisor to the Secretary. Brian organized the standup of the VA's Coordinating Council on National Health Reform and directed the activities of its multi-team Health Reform Working Group. He was also a member of the Institute of Medicine's Committee on the Governance and Financing of Graduate Medical Education.

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Jeff Allen, PhD

Jeff Allen, Ph.D. serves as the President and CEO of Friends of Cancer Research (Friends). During the past 20 years, Friends has been instrumental in the creation and implementation of policies ensuring patients receive the best treatments in the fastest and safest way possible. As a thought leader on many issues related to Food and Drug Administration, regulatory strategy and healthcare policy, he is regularly published in prestigious medical journals and policy publications, and has contributed his expertise to the legislative process on multiple occasions. Recent Friends initiatives include the establishment of the Breakthrough Therapies designation and the development of the Lung Cancer Master Protocol, a unique partnership that will accelerate and optimize clinical trial conduct for new drugs. Dr. Allen received his Ph.D. in cell and molecular biology from Georgetown University, and holds a Bachelors of Science in Biology from Bowling Green State University.



Gideon Blumenthal, MD

Dr. Gideon Blumenthal is a hematologist oncologist who is currently Vice President, Global Regulatory Affairs in Oncology, Merck. Prior to joining Merck, Dr Blumenthal spent over a decade at the US Food and Drug Administration Oncology office, taking on increasing leadership responsibilities during his time at the Agency. He initially served as a medical reviewer, then clinical team leader, followed by Acting Deputy Director in the Office of Hematology Oncology Products and Associate Director for Precision Oncology, and most recently served as the Deputy Center Director of the Oncology Center for Excellence. Dr Blumenthal did his internal medicine training at the University of Maryland School of Medicine, followed by a hematology oncology fellowship at the National Cancer Institute. He was an attending physician in the NCI thoracic oncology clinic. He received numerous awards, including the 2018 American Society for Clinical Oncology Public Service Award. He has co-authored over 90 articles in the Oncology and Drug Development peer reviewed literature and has authored 3 book chapters.

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Alicyn Campbell

Alicyn Campbell is currently Head of Digital Health Oncology R&D at AstraZeneca. Alicyn has over 12 years of experience in Health Outcomes Research. Most recently, she served as the Global Head of Patient Centered Outcomes Research at Genentech/Roche. In that capacity, she was responsible for leadership in the assessment of the patient experience and consulted widely with the FDA and international regulators. She achieved the first ever novel FDA patient reported outcome data approved in label for Hycela and was also responsible for novel patient-reported efficacy data for Hemlibra. She is the Founder, Executive Sponsor and Co-chair of Industry PRO-CTCAE Working Group, recognized as part of the 'Cancer Moonshot' initiative by former US Vice President Biden, and is a frequent research collaborator to Friends of Cancer Research and LUNGevity. She has also authored several significant scientific publications and presentations, latest in The Lancet Oncology.



Jonathan Hirsch

Jonathan Hirsch is the Founder and President of Syapse, a market leader in precision medicine and real world evidence solutions. Jonathan founded Syapse in 2008, working closely with healthcare providers, life sciences companies, molecular diagnostics labs, and government agencies to create products that improve patient outcomes through precision medicine. Jonathan's work includes catalyzing national cancer data sharing networks, including serving on the White House Cancer Moonshot Data Sharing Working Group, and chairing the Data Committee for GBM AGILE, a global initiative to find a cure for brain cancer. Before founding Syapse, Jonathan worked in neuroscience commercial development at Abbott Laboratories (now AbbVie). Jonathan received an MSci in Neuroscience from Stanford University and an AB in Biology and Political Philosophy from the University of Chicago.

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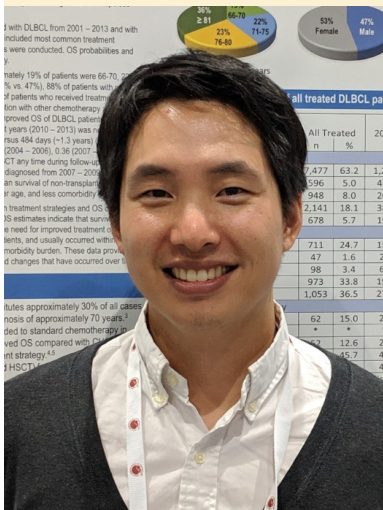


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Christopher Kim, PhD

An epidemiologist at Amgen in the Center for Observational Research (CfOR) where my team is focused on hematologic malignancies conducting real world evidence (RWE) studies to demonstrate unmet need, to provide context to single arm clinical trials by identifying external historical control groups, designing post-marketing safety commitment studies (PMC/PMR), evaluate risk management minimization plans (RMM/RMP), compare effectiveness of drugs/regimens, identify treatment trends, and contribute to understanding the epidemiology of various diseases for the development of molecules and drugs post-marketing authorization.

Prior to Amgen, formerly a post-doctoral fellow at National Institutes of Health/National Cancer Institute in the Division of Cancer Epidemiology and Genetics. Graduate from the Epidemiology and Public Health department at Yale University (PhD) and Biology department at Boston University (BA).

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SPEAKER BIOGRAPHIES

SESSION 4 | FROM BROOKLYN TO BEIJING: GLOBAL RARE DISEASE REGISTRIES AS GLOBAL LEARNING PLATFORMS



Amy P. Abernethy, MD, PhD

Amy P. Abernethy, M.D., Ph.D. is an oncologist and internationally recognized clinical data expert and clinical researcher. As the Principal Deputy Commissioner of Food and Drugs, Dr. Abernethy helps oversee FDA's day-to-day functioning and directs special and high-priority cross-cutting initiatives that impact the regulation of drugs, medical devices, tobacco and food. As acting Chief Information Officer, she oversees FDA's data and technical vision, and its execution. She has held multiple executive roles at Flatiron Health and was professor of medicine at Duke University School of Medicine, where she ran the Center for Learning Health Care and the Duke Cancer Care Research Program. Dr. Abernethy received her M.D. at Duke University, where she did her internal medicine residency, served as chief resident, and completed her hematology/oncology fellowship. She received her Ph.D. from Flinders University, her B.A. from the University of Pennsylvania and is boarded in palliative medicine.



Sean Khozin, MD, MPH

Sean Khozin, MD, MPH is the Global Head of Data Strategy at Janssen R&D, Johnson & Johnson and a research affiliate at MIT Laboratory for Financial Engineering. He joined Johnson & Johnson from US FDA's Oncology Center of Excellence, where he led the Center's bioinformatics efforts. He was also the founding Executive Director of Information Exchange and Data Transformation (INFORMED), US FDA's first data science and technology incubator established under special authorities from the US Department of Health and Human Services. INFORMED was designed as an agile sandbox for developing next-generation solutions through foundational research and public-private partnerships aimed at improving product development and advancing national public health priorities.

Prior to his tenure in federal government, Dr. Khozin was the cofounder of Hello Health, a technology company focused on developing integrated telemedicine, point-of-care data visualization, and advanced analytical solutions for optimizing patient care and clinical research. The company's core technology offerings were first operationalized in a multidisciplinary network of clinics called SKMD that Dr. Khozin had founded and served as Chief Medical Officer.

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Eric Marsh, MD, PhD

Dr. Marsh received his Medical Degree at New York University School of Medicine, as well as PhD in physiology and neuroscience at NYU Sackler School of Biomedical Sciences. He then completed his Internship and pediatric residency at NYU. He moved to CHOP for a Child Neurology residency where he stayed for fellowship in clinical neurophysiology and also completed a post doctoral fellowship, followed by joining the faculty at Perelman School of medicine in the Departments of Neurology and Pediatrics. He is now an associate professor of neurology at University of Pennsylvania and CHOP and recently became the Clinical Director at the Penn Orphan Disease center.

As a key member of the CHOP epilepsy program, and clinical director of the neurogenetics program, he has particular clinical interests in developmental epilepsies and cortical malformations. His research has focused strongly on the impact of intraneuronal development and altered excitability on epilepsy, as well as analyzing intracranial EEG recordings to better localize the epileptic zone and network. This work has primarily utilized a model of mutations in the gene ARX, but also performing basic science work with a number of other developmental genes including mouse work on Cdkl5. He has been involved in a number of clinical trials, being one of the first sites in the US to treat children with Cannabidiol and then being an active site for the industry sponsored clinical trials leading to approval of Epidiolex. He is actively involved in clinical trials for CDD, Rett syndrome, Dravet syndrome and other genetic developmental disorders. He is the site lead for the International Foundation for CDKL5 research Center of Excellence, Rett syndrome Research Center of Excellence, and Dravet Syndrome Center of Excellence at CHOP.



Elizabeth Powers, MBA

Elizabeth is Category Lead for regulatory and safety offerings in IQVIA's Real World Solutions division. Her responsibilities include applying advances in data and technology to post-marketing requirements with the aim for driving greater value for effort in evidence generation. She also serves as a strategic advisor to senior pharma executives around strategic use of real world evidence across the pharma enterprise and product lifecycle. She has over 15 years of experience in biotech and pharma, with a particular focus on the intersection of pharma and payers / HTA bodies and RWE. This includes experience in developing RWE strategies and data platforms. She has also led complex external comparator studies for gene therapies and rare diseases.

She has published articles in Harvard Business Review, InVivo and Financial Executive International. She holds a BA in mathematics from St. John's College, Annapolis and Santa Fe, and an MBA in Finance and Information Strategy from The Wharton School at University of Pennsylvania. She has published multiple articles on the topic of pharma engagement with payers and IDNs in InVivo and has published article an article on organizational effectiveness in Harvard Business Review.

Elizabeth earned an MBA from The Wharton School at the University of Pennsylvania.

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Steve Usdin

Steve has been BioCentury's Washington Editor since 1993, covering political and policy issues affecting the life sciences sector. He also is BioCentury's Senior Editor responsible for coverage of social issues involving biotechnology, as well as the former host of BioCentury This Week, BioCentury's public affairs television program broadcast in 2010-14.

Steve's reporting about biotechnology and biomedical policy has been cited in The Economist, The Wall Street Journal, The Washington Post, New Scientist, Nature, Politico and other publications. In 2012, the FDA Alumni Association named Steve the Harvey W. Wiley Lecturer, making him the first journalist to receive the Wiley Award.

Steve is also a widely recognized scholar on espionage and the Cold War. He is the author of Bureau of Spies: The Secret Connections between Espionage and Journalism in Washington (Prometheus Press, 2019) and Engineering Communism: How Two Americans Spied for Stalin and Founded the Soviet Silicon Valley (Yale Univ. Press, 2008).



Glen de Vries

Glen de Vries is co-CEO and co-founder of Medidata, the most-used platform for clinical trials around the world. Medidata has powered tens of thousands of clinical trials, with millions of patients and billions of patient records. Glen has been driving Medidata's mission since the company's inception in 1999, "powering smarter treatments and healthier people" by advancing pharmaceutical business transformation with technology, non-traditional ways of thinking, and industry collaboration. In 2019, in one of the largest health care technology acquisitions in history, Medidata became part of Dassault Systèmes. Glen's publications have appeared in Applied Clinical Trials, Cancer, The Journal of Urology, Molecular Diagnostics, and other journals. He serves as a trustee of Carnegie Mellon University, and is the author of The Patient Equation (de Vries & Blachman, 2020).

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Jill Weimer, PhD

Dr. Jill Weimer is a developmental neuroscientist and oversees the management and continued development of the translational arm of Sanford Research in Sioux Falls, South Dakota. She started at Sanford Research in 2009, and her research program focuses on the molecular mechanisms mediating development of the cerebral cortex and how disruption in these processes can lead to a whole host of neural pediatric disorders, including Batten's disease and Neurofibromatosis Type 1. Work in Dr. Weimer's lab help lead to the first ever gene therapy trial programs for CLN3 and CLN6 –Batten disease. In June 2019, Dr. Weimer joined the Amicus Therapeutics team as the Senior Vice President of Discovery Research and Gene Therapy Science. She plays a unique dual role holding leadership positions with both Sanford Research and Amicus, in addition to leading her research lab. Dr. Weimer grew up in north central Missouri and moved to upstate New York where she received her bachelor's degree and Ph.D. in neuroscience from the University of Rochester. She completed her postdoctoral training in the Neuroscience Research Center at the University of North Carolina in Chapel Hill with a focus on developmental neuroscience.

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